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Patient Involvement in Health Technology Assessment

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To Philip, Carl and Glenn for supporting us throughout this book's development

Foreword I

If you're not involving patients, you're not doing HTA!

It's that simple. Patient involvement improves the quality, relevance, and value of HTA. It is difficult to conceive of health technology assessment being conducted in a meaningful way in the twenty-first century without the involvement of patients.

As the President and CEO of a Health Technology Assessment agency, and as Chair of the International Network of Agencies for Health Technology Assessment (INAHTA), I am a strong advocate for patient involvement in HTA.

Patient Involvement in HTA explores the rationales underlying patient involvement, provides research methodologies to produce patient-based evidence, describes how patient involvement is being achieved in different countries, and provides the point of view of various stakeholders. They go one step further by providing insight into how all stakeholders can contribute to making patient involvement more robust and meaningful.

The book is truly an international collaboration with contributions from a virtual who's who of experts from INAHTA member agencies around the globe—not to mention patient organisations, academia, health technology developers, and health care funders.

Don't think you've done your part by buying this book. Read it. Learn from it. Let it spark discussions about what you, your colleagues, your organisation, and your networks can do to improve patient involvement in HTA. There are important messages for all stakeholders, and I will do my part to support and indeed implement many of the proposals identified in the book—within my agency and more broadly across INAHTA member agencies.

Facey, Single, and Hansen have given us an invaluable resource that can help us improve the quality and relevance of HTA. I encourage you to take up their call to action.

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Chair of the International Network of Agencies
for Health Technology Assessment (INAHTA)
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Foreword II

The development of new health technologies, be it at the scientific bench or the clinical bedside, involves the involvement of patients in one way or another. So, it seems profoundly intuitive that patient involvement also occurs during health technology assessment (HTA). However, the lack of meaningful ways for patients to participate and influence HTA is still very noticeable across the globe. This book places patient involvement in HTA within a strong scientific and policy context. It establishes a coherent case that the scientific basis of HTA is not robust without research to produce patient-based evidence and participation of patients in the HTA process. It describes credible methodologies to achieve it. By classifying the ways in which patients interact with HTA, providing their perspectives, identifying their preferences, and helping to uncover evidence uncertainties, the authors describe a starting point from which the HTA community can debate the opportunities and challenges of patient involvement. Everyone involved in HTA should read this book. It doesn't provide all the answers. But it will certainly stimulate those with a strong desire to ensure that HTA has face and construct validity to think what more they can, and should, do to incorporate patients' perspectives and experiences in their work.

Carole Longson
Past President, Health Technology Assessment international

Centre for Health Technology Evaluation,
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London, UK

Preface

The Birth of the Book

Can we assess the value of a health technology without involving patients? Many HTA bodies have no or only limited patient involvement in HTA. Their reasons are well documented: it's too expensive, it uses too many resources, it takes too long, it's too biased, it's hard to use in a scientific process, there is no established good practice, it lacks methods, it adds little, and it's just politics. While these concerns may be genuine, are they accurate or sufficient to overlook a potentially important source of information? Can we really know the value of most health technologies in the absence of knowledge of patients' needs, preferences and experiences?

As patient involvement has emerged and evolved in HTA, we have been challenged by others about its value and practical application. And perhaps because we come from diverse backgrounds (statistics, anthropology, stakeholder engagement) and different cultures (British, Danish, Australian), we've been challenging each other on these issues since we began exchanging ideas through Health Technology Assessment international (HTAi) more than a decade ago.

At the first HTAi annual meeting in Krakow in 2004, a panel session about patients' perspectives took place. It was the only place in the conference of HTA professionals, researchers and health technology developers where patient issues were mentioned. Karen M. Facey moderated the session, including presentations from Laura Sampietro-Colom and Helle Ploug Hansen. The discussion was lively and demonstrated the imperative for HTA to focus on patients and citizens. The following year, Karen and Laura established the HTAi Interest Group for Patient and Citizen Involvement in HTA. In 2008, at a pre-conference workshop, the Interest Group was challenged to explain what it meant by involvement—was it research into patients' perspectives or was it participation in the HTA process? After much reflection, in 2010, we published our answer in the paper 'Patients' perspectives in health technology assessment: A route to robust evidence and fair deliberation'. In it, we set out our belief that it was both research and participation, a definition informed by wider global involvement initiatives in policy but uniquely shaped by

healthcare and scientific tradition in HTA. That year, at the HTAi annual meeting in Dublin, patient issues began to gain attention in sessions outside those run by the Interest Group.

At the HTAi annual meeting in Washington in 2013, Timothy Wrightson from Springer Publishers told Karen M. Facey and Helle Ploug Hansen that something was missing. The HTAi society with its academics and HTA professionals lacked a book on patient involvement in HTA. Timothy asked if Karen and Helle could and would take the lead as editors on such a book. Like most people, Karen and Helle did not have the time to add a book to already heavy workloads, and they knew all too well how challenging a task it would be to bring together the diverse array of work in this field. They should decline. Instead, they discussed it with members of the HTAi Interest Group. Everyone thought the book was needed and several offered to write chapters, so Karen and Helle accepted the invitation from Springer. However, after creating the book's outline, contacting possible authors and reviewing abstracts, they realised that they needed one more person in the group of editors. So, Ann N.V. Single was contacted and accepted this challenge.

So why have we worked with more than 80 authors in five continents to produce this book? In truth, it was not just the gaping need for an academic text on this subject. We were also eager to spend time taking stock of the field, looking for answers in the many papers published and learning from the authors contributing chapters. Do we now have the research approaches? What are the best mechanisms to support patient participation? Can we use consistent terms and what do we mean by them? What are the consequences of our definition of patient involvement? How are HTA bodies involving patients? What are the implications of reduced budgets and rapid HTAs? Is patient involvement making a difference and how would we know? What needs to happen next? This book tries to give at least tentative answers to these questions.

Contributors

As HTA is interdisciplinary, it has been important to obtain different views about patient involvement in HTA. The contributors of this book come from a wide range of professions and positions in different organisations. They come from different countries and cultures, and authors are writing from their own perspectives and experiences. Although all of the authors manage the English language, they have faced many challenges on their writing journey, including unpacking the different meanings and practice behind common words and concepts and responding to insights found in other chapters. We have encouraged the authors to use a common language and style, creating consistency and continuity across the chapters to form a cohesive book that can be read from cover to cover, rather than a random selection of works. We do not necessarily agree with all the authors, but we welcome their views to challenge our assumptions and encourage debate and research in a field that is rapidly developing.

Since the beginning of 2015, the authors of the 37 chapters of this book have worked collaboratively with skill, thoughtfulness and tenacity together with their associates and the editors. Difficult questions have been asked and patience has been tested, but commitment has not waned. New ideas have been generated and existing ideas have been adapted to HTA, filling gaps in knowledge and competencies within patient involvement in HTA. Several authors had their own experiences of serious illness during the writing period and showed determination to complete their chapters. In particular, we offer our condolences to the family of Dr. Christin Andersson who passed away in December 2016.

Content and Organisation

The book is written for academics and HTA professionals. The conceptual, political and ethical rationales for patient involvement in HTA are explored in Part I, with specific research methodologies described in Part II. Part III then presents examples from a range of HTA bodies and stakeholders showing how patient involvement is achieved and plans for improving processes. It is our hope that this book will find its place in master's programmes, in PhD courses and in every HTA body. Furthermore, it is our hope that it will give those working in HTA the confidence to review the evidence for patient involvement and implement the methods that improve assessments.

Now, the book is here. We hope it will be 'a page turner'. At the same time, we hope you will return again and again to it to reach a deeper and more complex understanding of patient involvement in HTA that emboldens you to reflect and alter your own practice when involving patients.

Ashgrove, QLD, Australia — Ann N.V. Single
Edinburgh, UK — Karen M. Facey
Odense, Denmark — Helle Ploug Hansen

Acknowledgements

We recognise that patient involvement depends on patients and patient groups and wish to acknowledge the countless patients, relatives, caregivers and volunteers who take part in research or participate in HTAs. We are indebted to you.

Contents

Abbreviations

AGENAS	National Agency for Regional Health Services (Italy)
AHP	Analytic hierarchy process
AIDS	Acquired immune deficiency syndrome
AIFA	Italian Medicines Agency
AIJ	Aggregation of individual judgements
AIP	Aggregation of individual priorities
AR	Assessment report
BC	British Columbia
CADTH	Canadian Agency for Drugs and Technologies in Health (Canada)
CAT	Computer adaptive testing
CBA	Cost-benefit analysis
CDE	Center for Drug Evaluation (Taiwan)
CDR	Common Drug Review (Canada)
CEE	Central and Eastern Europe
CEO	Chief executive officer
CER	Comparative effectiveness research
CFA	Confirmatory factor analysis
CFM	Federal Council of Medicine (Brazil)
CHF	Consumers Health Forum of Australia
CI	Consistency index
CIHR	Canadian Institutes of Health Research
CMF	Comprehensive management framework
CNS	National Health Council (Brazil)
CONITEC	National Committee for Health Technology Incorporation into the SUS (Brazil)
CORD	Canadian Organization for Rare Disorders
COSMIN	Consensus-Based Standards for the Selection of Health Measurement Instruments
CR	Consistency ratio
CUA	Cost-utility analysis
D4D	Devices for Dignity

DACEHTA	Danish Centre for HTA
DBC	Drug Benefit Committee (Taiwan)
DCE	Discrete choice experiment
DoH	Department of Health
ECHTA	European Collaboration for HTA
EFA	Exploratory factor analysis
EFNA	European Federation of Neurological Associations
EMA	European Medicines Agency
EPF	European Patients' Forum
EU	European Union
EULAR	European League Against Rheumatism
EUnetHTA	European Network for Health Technology Assessment
EUPATI	European Patients' Academy on Therapeutic Innovation
EUR-ASSESS	Coordination and Development of Health Care Technology Assessment in Europe
EURORDIS	European Organisation for Rare Diseases
FDA	Food and Drug Administration
FROM	Family Reported Outcome Measure
G-BA	Federal Joint Committee
GRIPP	Guidance for Reporting Involvement of Patients and Public
HidroQOL	Hyperhidrosis Quality of Life Questionnaire
HIT	Headache Impact Test
HIV	Human immunodeficiency virus
HQO	Health Quality Ontario
HRQoL	Health-related quality of life
HST	Highly specialised technology
HTA	Health technology assessment
HTAi	HTA international
IAPO	International Alliance of Patients' Organizations
IJTAHC	International Journal of Technology Assessment in Health Care
IMI	Innovative Medicines Initiative
INAHTA	The International Network of Agencies for Health Technology Assessment
INESSS	Institut national d'excellence en santé et en services sociaux (Québec)
IQWiG	Institute for Quality and Efficiency in Healthcare
ISPOR	International Society for Pharmacoeconomics and Outcomes Research
IWG	Innovation Working Group (Italy)
MAPPs	Medicines Adaptive Pathways to Patients
MBS	Medicare Benefits Schedule (Australia)
MCDA	Multi-criteria decision analysis
MHRA	Medicines and Healthcare products Regulatory Agency
MIC	Minimal important change
MID	Minimal important difference

MoH	Ministry of Health
MRS	Marginal rate of substitution
MSAC	Medical Services Advisory Committee (Australia)
MTA	Multiple technology assessment
N/A	Not applicable
NHC	National Health Council (USA)
NHIA	National Health Insurance Administration (Taiwan)
NHS	National Health Service
NICE	National Institute for Health and Care Excellence (England and Wales)
NIHR	National Institute for Health Research (UK)
OHTAC	Ontario Health Technology Advisory Committee
OMERACT	Outcome Measures in Rheumatology
OPDP	Ontario Public Drug Programs
OTA	Office of Technology Assessment (USA)
PBAC	Pharmaceutical Benefits Advisory Committee (Australia)
PBRS	Pharmaceutical Benefits and Reimbursement Scheme
PBS	Pharmaceutical Benefits Scheme (Australia)
PCDT	Clinical Protocols and Therapeutic Guidelines (Brazil)
pCODR	pan-Canadian Oncology Drug Review
PCOR	Patient-centred outcomes research
PCORI	Patient-Centered Outcomes Research Institute
pERC	pCODR Expert Review Committee
PI	Patient involvement
PICO	Population, intervention, comparator, outcome
PIP	Public Involvement Programme (England)
PPRN	Patient-Powered Research Networks
PRO	Patient-reported outcome
PROM	Patient-reported outcome measure
PROMIS	Patient-Reported Outcomes Measurement Information System
PRP	Patient research partner
PsAID	Psoriatic Arthritis Impact of Disease Score
QALY	Quality-adjusted life-year
QES	Qualitative evidence synthesis
QOL	Quality of life
RCT	Randomised controlled trials
RETREAT	Review question, Epistemology, Time, Resources, Expertise, Audience and purpose, Type of data
RUM	Random utility maximisation
SBU	Swedish Agency for HTA and Assessment of Social Services
SDC	Smallest detectable change
SEED	Shaping European Early Dialogues
SHI	Statutory health insurance
SHTG	Scottish Health Technologies Group
SiHTA	Society for Italian HTA

SMC	Scottish Medicines Consortium
SPICE	Setting, Perspective, phenomenon of Interest, Comparison, Evaluation
SPOR	Strategy for Patient-Oriented Research
STA	Single technology assessment
SUS	Brazilian Public Health System
TA	Technology assessment
TDM	Tribunale per I Diritti del Malato (Italy)
TLV	Swedish Dental and Pharmaceutical Benefits Agency
TTO	Time trade-off
WCE	Wireless capsule endoscopy
WHO	World Health Organization
WTP	Willingness to pay

Editors' Biographies

Karen M. Facey, PhD, is an honorary research fellow at the University of Edinburgh, chartered statistician, honorary member of the Faculty of Public Health and fellow of the Royal Society of Medicine. She has worked as a statistician for pharmaceutical companies and the UK Medicines Regulatory Agency. In 2000, she set up the first national health technology assessment (HTA) agency in Scotland and since 2003 has been an independent consultant. She has been active in HTA international (HTAi) for the past decade, chairing its Policy Forum, and was founding chairperson of the HTAi Interest Group for Patient and Citizen Involvement in HTA. She is on the editorial board of several journals including *The Patient* and *Research Involvement and Engagement*. She has research interests in HTA policy, patient involvement and rare diseases. In 2014, she was named as one of the top 100 practising scientists in the UK for her work on HTA and patient well-being.

Helle Ploug Hansen, PhD MA RN, is professor in humanistic rehabilitation research, the University of Southern Denmark, Department of Public Health, Research Unit of General Practice, Denmark. She holds a PhD degree and an extended master's degree in anthropology from the University of Copenhagen. She has been active within the field of HTA since 2001 and among other things been the author of several chapters in the *Danish Handbook of HTA*. Furthermore, Dr Hansen has edited a special issue in the *International Journal of Technology Assessment in Health Care* together with Karen Facey. She has conducted several ethnographic fieldwork studies in Denmark addressing psycho-social and rehabilitative aspects related to men and women with cancer. She has published several books and many peer-reviewed articles. She is visiting professor in nursing at a rehabilitation hospital in Oslo, Norway.

Ann N.V. Single, BBus (journalism), MA (writing), specialises in patient involvement and communication. She has been an active member of the HTAi Interest Group for Patient and Citizen Involvement in HTA for the past decade and a lay reviewer for several journals. She has previously worked as the director of

communication and patient involvement at the Health Technology Board for Scotland, managed science engagement programmes in Australia and served as secretary for a patient group. She has contributed to a variety of papers on patient involvement and tools for patient participation including compiling a glossary of HTA terms for patients. She is interested in storytelling, especially what can be learnt from the stories of patients and caregivers.

Part I
Conceptualisation

Chapter 1
Health Technology Assessment

Karen M. Facey

1.1 Introduction

This chapter will explore the history of health technology assessment (HTA) and how it has evolved in terms of processes and methods, highlighting elements relevant to patient involvement to lay a foundation for the subsequent chapters of this book. HTA is a policy analysis that seeks to inform decision-makers in national, regional or hospital health services about the use of health technologies. HTAs require systematic processes that critically assess research about the impacts of using the health technology along with context-specific appraisal of the social, economic, legal and ethical implications of the use of the health technology. This is not simply a scientific endeavour. It requires interdisciplinary deliberative discussion and value judgements about the relevance of the evidence for the local health system. HTAs may recommend the use or disinvestment of a health technology and so are subject to political, public and stakeholder scrutiny. As a result there has been pressure to involve those who have a specific interest in the health technology, particularly patients, in the HTA process. However, this is contentious due to concerns about potential bias and representativeness of patient input and the scientific integrity of patient evidence.

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1.2 Context and History

Health systems, whether funded by taxation, social insurance, personal insurance or private fees, need to organise their services to use available funds efficiently to deliver effective, safe, person-centred care in a timely and equitable manner for the population they serve (Committee on Quality Health Care in America 2001). They have to make choices about who to treat, with what intervention, in what setting and for how long (Newdick 2004). Such questions must be considered not just in the context of the individual patient but in terms of providing the best possible service to all potential users of the health system (Drummond et al. 2006, Chap. 2). Thus resource allocation questions often seek to maximise health gain of the population overall, recognising that there is an opportunity cost to any investment (giving up the possibility of funding an alternative intervention with that money) (Metzler and Smith 2012).

Daniels and Sabin (2008 [1]) stated that resource allocation decisions in healthcare were 'rife with moral disagreements and a fair, deliberative process is necessary to establish legitimacy and fairness of such decisions'. They argued that resource allocation:

1. Processes must be public (fully transparent) about the grounds for decisions.
2. Decisions must rest on reasons that stakeholders can agree are relevant.
3. Decisions should be revisable in light of new evidence and arguments.
4. Should include assurance, through enforcement, that these three conditions are met.

In fact, three decades earlier, the US Senate had noted that 'a reasonable amount of justification should be provided before costly new medical technologies and procedures are put into general use' (Office of Technology Assessment OTA 1976 [vii]). As a result, the OTA created a report providing examples of medical technologies in the fields of diagnostics, implantable devices, vaccines, surgery, medicines and interventional procedures that illustrated the diversity in development, purpose and the use of medical technologies (OTA 1976). It noted that decisions about the use of such new technologies were often made on the basis of evidence about technical feasibility, safety and anticipated need or demand, but that wider consideration of impacts should be assessed, including implications for:

- Patients
- Patients' families
- Society as a whole (environmental impacts, ethics, cultural values)

- Medical care system
- Legal and political systems
- The economy

OTA (1976) stated that to systematically consider these wider impacts of medical technologies, a comprehensive form of policy research was needed to provide decision-makers with policy alternatives. The formal process of 'technology assessment', which had first been used to evaluate other forms of technologies in 1965, was suggested. Technology assessment was described as systematically examining the short- and long-term social consequences (e.g. societal, economic, ethical, legal) of the application or use of technology, considering unintended, indirect or delayed social impacts (OTA 1976).

OTA (1976) described the unique features of technology assessment as being:

- Based on an explicit analytic framework, specified in advance
- Comprehensive in scope, examining impacts on social, ethical, legal and other systems that may not be immediately obvious
- Carried out by a multidisciplinary group
- Able to explicitly identify the groups that would be affected by the technology and evaluate the impacts (and impacts of impacts) of the technology on each party

The report (OTA 1976) outlined a list of questions to be considered for each potential area of impact. Box 1.1 shows the questions about the implications for patients and families of a new heart valve.

Box 1.1: Questions to Assess the Impacts of Medical Technologies on Patients: Heart Valve Example (OTA 1976, reformatted)

What are the implications of the technology for the patient?

What will be the quality of life of the patient who has been treated? Normally active?

Moderately restricted? Physically disabled?

A recipient of an artificial heart could reasonably expect to lead an active, productive, fairly normal life.

What psychological effects can be anticipated? Guilt? (Because of high financial and social costs to family) Anxiety? Feelings of dehumanisation? Dependency? Anxieties and even psychoses might be precipitated in heart recipients who are preoccupied by dependence on an inorganic source of power. Such reactions have been observed in patients receiving dialysis for chronic kidney disease. Furthermore, some of the drugs that might be used as supportive therapy, e.g. steroids, themselves have psychotropic effects.

If nuclear-powered artificial hearts are used, it may be necessary to identify or even monitor movement of recipients in order to protect the nuclear fuel and to recover it after death. Recipients might be required to waive some of the individual freedom most of us take for granted.

Will regimentation result from the use of the technology? Loss of freedom over one's body?

Death from heart disease is sometimes, although not always, swift and painless. Although the benefits of prolonging life with an artificial heart are obvious, the recipient will have to be made aware of the possibility of death from failure of the implant procedure.

Will the use of the technology increase the probability of a lingering and painful death?

Once surgery is complete, the procedure can be reversed only by removing or deactivating the artificial heart, thereby allowing the patient to die.

Will the effects of the new technology be reversible if the patient feels that its benefits are outweighed by its drawbacks? Will the individual be able to choose to die?

What are the implications for the patient's family?

Implantation of an artificial heart will permit survival of the patient, and the benefits to the rest of the family will be numerous. On the other hand, unless the cost of implantation of the heart is covered by some third-party payer, the enormous financial burdens could impoverish the patient's entire family and strain intrafamily relationships.

What will be the costs to the family? How will the new technology affect family structure?

The plutonium contained in a nuclear-powered artificial heart may, however well shielded, emit radiation that could pose some danger to family members who are frequently close to the patient.

Will there be any physical dangers to the immediate family?

Will the device or procedure be psychologically acceptable to the family?

Will active cooperation or assistance of family members be necessary on a continuing basis?

How will the new technology affect individual or family budgets? What purchases will families forego if they have to pay for the new technology?

1.3 Development of HTA

1.3.1 Spread of HTA

Although OTA was criticised as being an 'unnecessary agency' (Banta 2009 [8]) and closed in 1995, it stimulated technology assessment activities in other countries (Banta 2009). In Denmark, in 1982, and Sweden in 1987, national organisations were given responsibility to undertake systematic assessments of all forms of health intervention (including medical technologies, educational programmes, organisation of care) to inform policy and practice (Sigmund and Kristensen 2009; Jonsson 2009). So the ethos and processes of OTA's technology assessment were used under the new name of health technology assessment, with definitions of HTA that were taken directly from OTA's work. These have stood the test of time and have now been adopted by international societies and networks (Box 1.2).

Box 1.2: Definitions

Health technology is the application of scientific knowledge in healthcare and prevention, including technologies such as diagnostics, treatments, medical equipment, pharmaceuticals, rehabilitation, prevention methods, organisational and supportive systems within which healthcare is provided.

HTA is a multidisciplinary process that summarises information about the medical, social, economic and ethical issues related to the use of a health technology in a systematic, transparent, unbiased, robust manner. Its aim is to inform the formulation of safe, effective, health policies that are patient focused and seek to achieve best value. Despite its policy goals, HTA must always be firmly rooted in research and the scientific method.

(EUnetHTA 2016a)

HTA is a field of scientific research to inform policy and clinical decision-making on the introduction and use of health technologies. Health technologies include pharmaceuticals, devices, diagnostics, procedures and other clinical, public health and organisational interventions.

HTA is a multidisciplinary field that addresses the clinical, economic, organisational, social, legal and ethical impacts of a health technology, considering its specific healthcare context as well as available alternatives. The scope and methods of HTA may be adapted to the needs of a particular health system, but HTA processes and methods should be transparent, systematic and rigorous. In health systems throughout the world, HTA plays an essential role in supporting decision-making.

(Health Technology Assessment International (HTAi) 2016)

The proliferation of HTA in the past two decades is shown by the breadth of membership in the International Network of Agencies for HTA (INAHTA). INAHTA is a society for non-profit HTA bodies, with 51 members in 31 countries in 5 continents (INAHTA 2016). Furthermore, in 2014, the World Health Organization (WHO) issued a declaration recognising that HTA offers rigorous and structured research methodologies and transparent and inclusive processes. It recommended the use of HTA in all its member states to guide policies for rational and efficient use of medicines and devices to inform policies of universal health coverage and support sustainable health systems (World Health Organisation 2014).

1.3.2 The HTA Process

HTA has a scientific basis, involving critical appraisal of evidence available from research. However, as HTA seeks to inform policy about the use or organisation of a health intervention in a national, regional or local context, the process of doing HTA is different in each healthcare system. It takes account of the system's responsibilities, structure, care pathways and policy drivers (Chap. 31).

HTA has been described as:

- Assessment: critical review and scientific summary of the (international) evidence about relevant aspects of the health technology (Garrido et al. 2008)
- Appraisal: wider consideration of the assessment information, taking account of (local) values and other factors (Garrido et al. 2008)

The processes for assessment have been developed over decades and arise from the evidence-based medicine movement. However, HTA goes beyond evidence-based medicine to interpret the evidence in relation to the local healthcare system. For this interpretation, a multi-stakeholder advisory group is often needed. In HTA systems that feed directly into healthcare decisions (such as for reimbursement decisions), this process is called appraisal, but in other systems that are more advisory in nature, this terminology may not be used (Chap. 28).

In the past decade, it has also been recognised that HTA may need to extend its remit beyond traditional assessment/appraisal boundaries to influence the generation of evidence for a health technology over its life cycle of development and use (Facey et al. 2015). HTA bodies can provide helpful advice on clinical studies that are primarily designed for other purposes (such as a regulatory authorisation) or on research specifically commissioned to study particular implications of the health technology (such as comparative effectiveness, economic evaluations or user attitudes). Where there are major uncertainties in the evidence at the time of HTA assessment, further evidence collection may be instigated to collect specific outcomes to confirm the value of a promising health technology in the so-called managed entry agreements (Klemp et al. 2011). HTA bodies can advise in both situations on the evidence that would be of value to HTA.

1.3.3 HTA Methods

HTA is founded on scientific research and seeks to answer clear, structured research questions about the implications (direct and indirect, intended and unintended) of using the health technology. It is often structured using the PICO framework from evidence-based medicine (Sackett et al. 1997):

- **P**opulation (who should be treated)
- **I**ntervention (technical specification of health technologies under study, how they will be given)
- **C**omparator (health technologies currently used in the health service)
- **O**utcome (what outcomes/impacts are important)

Research questions are answered primarily by secondary research (systematic review of published literature with critical assessment of relevant studies) or if no literature can be found, by primary research (undertaking new research).

One of the first detailed HTA handbooks published in English came from the Danish Centre for HTA (DACEHTA) in 2001. It was updated in 2007 (Kristensen and Sigmund 2008) and presented a comprehensive model of HTA based on:

- Clinical effectiveness
- Cost effectiveness organisational issues
- Patient aspects

The handbook covered the planning of HTA, ethical considerations, systematic literature review (for all aspects of the HTA), primary research to understand stakeholders perspectives (qualitative methods, survey methods, analysis of registries and measurement of health status), clinical effectiveness, patient aspects, organisational issues, economic issues, synthesis and quality assurance to formulate a sound basis for decision-making.

In the 1990s and early 2000s, collaborative HTA work was undertaken among HTA bodies in the EUR-ASSESS[1] and ECHTA[2] Projects (Banta et al. 1997, Jonsson et al. 2002). This was followed in 2006, by the European Commission-funded project to develop a European network for HTA (EUnetHTA) and three subsequent Joint Actions[3] of European Union (EU) Member States. The centrepiece of this work from a methodological standpoint has been the HTA Core Model® (EUnetHTA 2016b), which has nine domains:

1. Health problem and current health technologies
2. Description/technical characteristics
3. Safety
4. Clinical effectiveness

[1] Coordination and Development of Health Care Technology Assessment in Europe

[2] European Collaboration on HTA

[3] Joint Actions are initiatives that are co-funded by the European Commission and Member States.

5. Costs/economic evaluation
6. Ethical analysis
7. Organisational aspects
8. Patient and social aspects
9. Legal aspects

The HTA Core Model® documentation is a detailed report of over 400 pages, which describes how each domain should be studied, including assessment elements (research questions) that might be relevant for each domain and methods to study those questions, by secondary or primary research.

Another handbook for HTA by Goodman (2014), based on his HTA 101 course, includes methodology chapters and policy topics that have emerged over the past decade including comparative effectiveness research, managed entry agreements (risk-sharing schemes, patient access schemes), innovation and rapid HTA.

These forms of HTA that study a range of impacts of a health technology are often called 'full HTA' or 'comprehensive HTA'.

Although the focus and methods used for HTA in each country vary, all HTA bodies evaluate the clinical effectiveness of a technology, assessing clinical evidence from international trials in relation to the clinical pathways in their local healthcare system. They are seeking to understand the 'added value' of a health technology compared to their current standard of care. Often, added value is not clear for the entire population, and so a specific sub-group may be identified in whom the added value is higher or who have greater need for a new technology because they have more limited alternatives.

Many HTA bodies also evaluate economic considerations such as cost-effectiveness (value for money) and budget impact (total cost per year of the treatment for all the patients that are expected to receive the treatment). Only a few HTA bodies systematically and explicitly evaluate social, legal or ethical issues or organisational or patient aspects.

One of the major changes to HTA methodology in the 2000s was the move away from comprehensive HTAs to more rapid processes that could inform reimbursement/coverage decisions of medicines. This has meant HTAs occur at the point of market launch when the only evidence available is from the clinical research developed for the regulatory submission, which may not be published (Facey et al. 2015).

To inform reimbursement, HTAs had to be produced much quicker and in larger numbers than comprehensive HTA allowed. These more rapid HTAs also required new processes that were less resource intensive for HTA staff. So there was a move away from HTA researchers undertaking systematic reviews of all published evidence and producing comprehensive reports about all the implications of using a health technology. Instead, submissions of evidence were sought from health technology developers, or rapid literature reviews of other systematic reviews were undertaken. This has resulted in shorter HTA reports targeted at decision-makers (Watt et al. 2008). In the past decade, as new countries have instigated HTA, most have taken on these more rapid processes, and so assessment of the wider implications of using a health technology have been lost (Nielsen et al. 2011).

To inform reimbursement, HTAs had to be produced much quicker and in larger numbers than comprehensive HTA allowed. These more rapid HTAs also required new processes that were less resource intensive for HTA staff. So there was a move away from HTA researchers undertaking systematic reviews of all published evidence and producing comprehensive reports about all the implications of using a health technology. Instead, submissions of evidence were sought from health technology developers, or rapid literature reviews of other systematic reviews were undertaken. This has resulted in shorter HTA reports targeted at decision-makers (Watt et al. 2008). In the past decade, as new countries have instigated HTA, most have taken on these more rapid processes, and so assessment of the wider implications of using a health technology have been lost (Nielsen et al. 2011).

HTAs often find 'uncertainties' in the evidence due to:

- Short-term outcomes studied in controlled clinical trials that may not reflect clinical practice
- Lack of data about the health technology comparator of interest
- Limited evidence about the costs and impacts of the health technology and its comparator over the lifetime of a patient

These are often exacerbated in rapid HTAs, where evidence is only available from limited sources over a short time period.

These uncertainties are often the key areas of discussion by an appraisal or multi-stakeholder advisory committee. Indeed, Hofmann noted that value judgements are needed in economic evaluations, analysis of ethical, legal and social issues and reporting of HTA results and in appraisal and decision-making (Hofmann et al. 2014). However, few HTA bodies are explicit about their scientific and social value judgements (Rawlins 2014, Hofmann et al. 2014).

As (OTA 1976) identified, when there may be differences in values, as broad a group as possible should be involved in preparing the assessment, including adversaries on certain issues. Daniels and Sabin (2008) also indicated that decisions must rest on reasons that stakeholders can agree. Furthermore, as HTAs have been increasingly used to inform reimbursement/coverage decisions that manage patient access to health interventions, public and patient interest in HTA has increased. As a result, various groups of stakeholders and academics have developed principles for HTA (Wilsdon and Serota, 2011) that cover the structure, methods, process and use of HTA. These all include the need to involve stakeholders in HTA.

1.4 Patient Involvement in HTA

HTA processes could be considered as including the five pillars of quality relating to effectiveness, safety, efficiency, timeliness and equity, and so the sixth pillar of quality relating to patient centredness should also be included (Committee on Quality Health Care in America 2001). Moreover, it has now been recognised that patient involvement in HTA can contribute to democratic, technocratic, scientific and instrumental goals (OHTAC Public Engagement Subcommittee 2015).

In this chapter (and for the rest of the book), we use the term ‘patient’ to mean anyone who has direct experience of living with the condition being studied in the HTA or who may be eligible to receive the technology (e.g. specific members of the public who might be invited for vaccination or to undertake a diagnostic intervention). This can include individuals who have had or have the condition, informal caregivers (sometimes called carers) and voluntary groups that advocate for patients, such as patient organisations, self-help groups, user groups and patient associations. This does not include general members of the public or citizens who may use other services in the health system or someone who is a clinical expert.

‘Involvement’ is a term that is used widely but may be understood in different ways in different countries and alternative terms such as engagement, participation and empowerment may be used (Barello et al. 2014). This book presents the concept developed by the HTAi Interest Group for Patient and Citizen Involvement in HTA (the HTAi Interest Group) that patient *involvement* in HTA encompasses two distinct but complementary ways in which HTAs could be strengthened by taking account of patients’ perspectives (adapted from Facey et al. 2010):

- *Research* into patient aspects (patients’ experiences, preferences, perspectives)
- Patient *participation* in the HTA process

Coulter (2004) stressed that as HTA involves value judgments, it should have greater patient and public participation. She stated that a patient-focused HTA would determine the types of questions that patients want to be answered and engage them in determining HTA priorities, designing and conducting assessments and appraisals, receiving and using findings from HTA and debating policy priorities and rationing. In 2010, Gauvin et al. (2010) provided a framework to consider the different levels of patient and public participation that could be used at every stage of HTA. This framework has been developed further in Chap. 5 to identify specific mechanisms of participation that have been used by the HTA bodies who present their work in Part III.

Coulter (2004) also stated that the HTA research process should include a variety of methods to determine the experience, views and preferences of wide groups of patients. The DACEHTA Handbook on HTA (Kristensen and Sigmund 2008) and the EUnetHTA Core Model® (EUnetHTA 2016b) presented methods to obtain robust evidence about patients’ perspectives and experiences, but this is within the context of the full HTA. For the many HTA bodies that focus on the assessment of clinical and cost-effectiveness, and who must do this in a rapid time frame, there are questions about how to develop robust patient-based evidence (Chap. 4). Like all issues in HTA, planning is key and such research should be planned well in advance (Facey et al. 2010) and international, multidisciplinary collaborations encouraged. Part II of this book will present methodologies for qualitative and quantitative research to understand patient aspects, including discussion of these challenges.

In 2014, HTAi undertook an international Delphi process to create consensus on *Values and Quality Standards for Patient Involvement in HTA* (HTAi 2014) as presented in Table 1.1 and Box 1.3. The values clearly relate to either research or participation, but the quality standards may relate more to participation with the assumption that research has its own ethical standards.

Table 1.1 HTAi Values for Patient Involvement in HTA (HTAi 2014)

Value	Descriptor
Relevance	Patients have knowledge, perspectives and experiences that are unique and contribute to essential evidence for HTA.
Fairness	Patients have the same rights to contribute to the HTA process as other stakeholders and have access to processes that enable effective engagement.
Equity	Patient involvement in HTA contributes to equity by seeking to understand the diverse needs of patients with a particular health issue, balanced against the requirements of a health system that seeks to distribute resources fairly among all users.
Legitimacy	Patient involvement facilitates those affected by the HTA recommendations/decision to participate in the HTA, contributing to the transparency, accountability and credibility of the decision-making process.
Capacity building	Patient involvement processes address barriers to involving patients in HTA and build capacity for patients and HTA organizations to work together.

Box 1.3: HTAi Quality Standards for Patient Involvement in HTA (HTAi 2014)

General HTA process

1. HTA organisations have a strategy that outlines the processes and responsibilities for those working in HTA and serving on HTA committees to effectively involve patients.
2. HTA organisations designate appropriate resources to ensure and support effective patient involvement in HTA.
3. HTA participants (including researchers, staff, HTA reviewers and committee members) receive training about appropriate involvement of patients and consideration of patients' perspectives throughout the HTA process.
4. Patients and patient organisations are given the opportunity to participate in training to empower them so that they can best contribute to HTA.
5. Patient involvement processes in HTA are regularly reflected on and reviewed, taking account of the experiences of all those involved, with the intent to continuously improve them.

For individual HTAs

6. Proactive communication strategies are used to effectively reach, inform and enable a wide range of patients to participate fully in each HTA.
7. Clear timelines are established for each HTA with advance notice of deadlines to ensure that appropriate input from a wide range of patients can be obtained.
8. For each HTA, HTA organisations identify a staff member whose role is to support patients to contribute effectively to HTA.
9. In each HTA, patients' perspectives and experiences are documented, and the influence of patient contributions on conclusions and decisions is reported.
10. Feedback is given to patient organisations who have contributed to an HTA, to share what contributions were most helpful and provide suggestions to assist their future involvement.

1.5 Discussion

As Part III of this book shows, HTA bodies vary widely in their roles and functions. Some undertake comprehensive HTAs; others perform rapid HTAs. Some have a remit to do the assessment, others do appraisal, some do both. Some assess individual health technologies in each HTA. Some assess a wide range of health technologies for a condition in one HTA. Some provide scientific advice to health technology developers about their trial design, and some manage registries to collect evidence post HTA to inform a future reassessment.

HTA appraisal committees judge the available evidence within the local social and political context, trying to create fair processes with consistent decisions that can be explained. As Coulter (2004) noted the balancing act of individual needs versus population requirements cannot be left to 'experts' alone. Patients (and citizens) need to understand the choices confronting policymakers and have the chance to be involved in determining priorities and trade-offs, but this must be done in a manner that promotes fair decisions for all users of the health system (Coulter 2004). Indeed, as Menon et al. (2015) stated, patient involvement can help resolve the decision uncertainties that arise in any HTA.

Patient involvement in HTA can help with the difficult value judgments that arise when clinical and economic evidence is limited, or added value is at the cusp of a pre-defined threshold, by explaining the real-world implications for patients. This becomes increasingly important as expedited regulatory pathways (Eichler et al. 2015, Food and Drug Administration 2015), an increased number of products for rare diseases and stratified medicine yield smaller clinical evidence bases. It is also relevant for all forms of health technologies other than medicines, where the evidence base has always been sparser.

HTA has been described as 'a bridge between the world of research and the world of decision-making' (Battista and Hodge 1999 [1464]). I have often modified this image to explain that patient involvement provides the lights on the bridge. It can alter the value judgments made in any HTA by elucidating the unintended and indirect impacts of the health technology, illuminating areas of unmet need, outcomes that matter to patients and informing determination of added value.

1.6 Conclusion

This chapter began with a review of HTA showing that when it was developed 40 years ago, it was intended to assess all the implications of using a health technology, and explicit questions were developed for patients and families. As HTA has evolved and been used to inform reimbursement and coverage decisions, comprehensive assessments are less common, and in many jurisdictions, focus has been placed on clinical effectiveness and cost-effectiveness. Systematic research on patients' perspectives and experiences has often been replaced by processes to help patients participate in HTA. These elements of research into patient aspects and patient participation are complementary, and both are the basis for how HTAi would define patient involvement in HTA. This is important because patient involvement

can identify unique patients' perspectives that can help interpret the clinical evidence base and inform the value judgments that are inherent throughout the HTA process.

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References

Banta HD, Werkö L, Cranovsky R, et al. Introduction to the EUR-ASSESS project. Int J Technol Assess Health Care. 1997;13:133–43.

Banta D, Jonsson E. History of HTA: Introduction. International Journal of Technology Assessment in Health Care. Cambridge University Press. 2009;25(S1):1–6.

Barello S, Graffigna G, Vegni E, Bosio AC. The challenges of conceptualizing patient engagement in health care: a lexicographic literature review. J Participat Med. 2014;6:e9.

Battista RN, Hodge MJ. The evolving paradigm of health technology assessment: reflections for the millennium. CMAJ. 1999;160:1464–7.

Committee on Quality Health Care in America. Crossing the quality chasm: a new health system for the 21st century. Washington DC: Institute of Medicine; 2001.

Coulter A. Perspectives on HTA: response from the patient's perspective. Int J Technol Assess Health Care. 2004;20:92–6.

Daniels N, Sabin JE. Accountability for reasonableness: an update. BMJ. 2008;337:a1850. doi:10.1136/bmj.a1850.

Drummond MF, Sculpher MJ, Torrance GW, O'Brien BJ, Stoddart GL. Methods for the economic evaluation of health care programmes. 2nd ed. Oxford: Oxford University Press; 2006.

Eichler H-G, Baird LG, Barker R. From adaptive licensing to adaptive pathways: delivering a flexible life-span approach to bring new drugs to patients. Clin Pharm Ther. 2015;97:234–46.

EUnetHTA. Common Questions. http://www.eunethta.eu/about-us/faq#t287n73 (2016a). Accessed 25 Jan 2016.

EUnetHTA. EUnetHTA joint action 2, work package 8. HTA Core model® version 3.0 (pdf). Copenhagen: EUnetHTA; 2016b. http://eunethta.eu/sites/5026.fedimbo.belgium.be/files/HTACoreModel3.0.pdf. Accessed 11 Apr 2016

Facey K, Boivin A, Gracia J, Hansen HP, Lo Scalzo A, Mossman J, et al. Patients' perspectives in HTA: a route to robust evidence and fair deliberation. Int J Technol Assess Health Care. 2010;26:334–40.

Facey K, Henshall C, Sampietro-Colom L, Thomas S. Improving the effectiveness and efficiency of evidence production for health technology assessment. Int J Technol Assess Health Care. 2015;31:201–6.

Food and Drug Administration. Fast track, breakthrough therapy, accelerated approval, priority review. Washington DC: FDA; 2015. http://www.fda.gov/forpatients/approvals/fast/ucm20041766.htm. Accessed 9 May 2015.

Garrido MV, Zentner A, Busse R. Health systems, health policy and health technology assessment. In: Garrido MV, Kristensen FB, Nielsen CP, Busse R, editors. Health technology assessment and health policy-making in Europe. Copenhagen: World Health Organisation on behalf of European Observatory on Health Systems and Policies; 2008. p. 53–78.

Gauvin F-P, Abelson J, Giacomini M, Eyles J, Lavis JN. "it all depends": conceptualizing public involvement in the context of health technology assessment agencies. Soc Sci Med. 2010;70:1518–26.

Goodman CS. HTA 101 – an introduction to health technology assessment. Bethesda MD: National Library of Medicine; 2014. https://www.nlm.nih.gov/nichsr/hta101/HTA_101_FIN. Accessed 5 Apr 2016

Hofmann B, Cleemput I, Bond K, et al. Revealing and acknowledging value judgments in health technology assessment. Int J Technol Assess Health Care. 2014;30:579–86. doi:10.1017/S0266462314000671.

HTAi. HTAi values and quality standards for patient involvement in HTA. (2014.) http://www.htai.org/fileadmin/HTAi_Files/ISG/PatientInvolvement/v2_files/Info/PCISG-Info-PosterValuesandStandards-30-Jun14.pdf. Accessed 31 Oct 2016.

HTAi. What is HTA? http://www.htai.org/htai/what-is-hta.html. (2016.) Accessed 25 Jan 2016.

INAHTA. International network of agencies for HTA. (2016). http://www.inahta.org/. Accessed 4 May 2016.

Jonsson E. History of health technology assessment in Europe. Int J Technol Assess Health Care. 2009;25(Supplement 1):42–52. doi:10.1017/S0266462309090412.

Jonsson E, Banta HD, Henshall C, Sampietro-Colom L. Special section: European collaboration for health technology assessment – developing an assessment network. Executive summary of the ECHTA/ECAHI project. Int J Technol Assess Health Care. 2002;18:213–7. doi:10.1017/S0266462302000235.

Klemp M, Frønsdal KB, Facey K. What principles should govern the use of managed entry agreements? Int J Technol Assess Health Care. 2011;27:77–83.

Kristensen FB, Sigmund HP. Health technology assessment handbook 2007 (2nd edition in English). Copenhagen: National Board of Health; 2008. http://sundhedsstyrelsen.dk/~/media/ECAAC5AA1D6943BEAC96907E03023E22.ashx. Accessed 5 Apr 2016

Menon D, Stafinski T, Dunn A, Short H. Involving patients in reducing decision uncertainties around orphan and ultra-orphan drugs: a rare opportunity? Patient. 2015;8:29–39. doi:10.1007/s40271-014-0106-8.

Metzler DO, Smith PC. Theoretical issues relevant to the economic evaluation of health technologies. In: Pauly MV, TG MG, Barros PP, editors. Handbook of health economic, vol. 2 ; 2012. Amsterdam (Oxford): North Holland (Elsevier)

Newdick C. Who should we treat? Rights, rationing and resources in the NHS. 2nd ed. Oxford: Oxford University Press; 2004.

Nielsen CP, Funch TM, Kristensen FB. Health technology assessment: research trends and future priorities in Europe. J Health Serv Res. 2011;16(Suppl 2):6–15.

Office of Technology Assessment (OTA). Development of medical technology – opportunities for assessment. Washington DC: United States Congress; 1976.

OHTAC Public Engagement Subcommittee. Public engagement for health technology assessment at health quality Ontario – final report from the Ontario health technology advisory committee public engagement subcommittee [internet]. Toronto: Queen's Printer for Ontario; 2015. http://www.hqontario.ca/Portals/0/documents/evidence/special-reports/report-subcommittee-20150407-en.pdf. Accessed 27 Aug 2016

Rawlins MD. Evidence, values, and decision making. Int J Technol Assess Health Care. 2014;30:233–8. doi:10.1017/S02664623140000154.

Sackett DL, Richardson WS, Rosenberg W, Haynes RB. Evidence based medicine – how to practice and teach EBM. New York: Churchill Livingstone; 1997.

Sigmund H, Kristensen FB. Health technology assessment in Denmark: strategy, implementation and developments. Int J Technol Assess Health Care. 2009;25(Supplement 1):94–101. doi:10.1017/S0266462309090485.

Watt A, Cameron A, Sturm L, Lathlean T, Badidge W, Blamey S, et al. Rapid vs full systematic reviews: an inventory of current methods and practice in health technology assessment. Int J Technol Assess Health Care. 2008;24:133–9.

Wilsdon T, Serota A. A comparative analysis of the role and impact of HTA – prepared for EFPIA, PhRMA, medicines Australia and EuropaBio. Charles River associates. Project no. D15891–00. 2011. http://www.efpia.eu/uploads/Modules/Documents/hta-comparison-report-updated-july-26-2011-stc.pdf Accessed 12 Apr 2016.

World Health Organisation. Health intervention and technology assessment in support of universal health coverage. WHO Declaration WHA67.23. Agenda Item 15.7.N24 2014. http://apps.who.int/gb/ebwha/pdf_files/WHA67/A67_R23-en.pdf?ua=1. Accessed 31 Oct 2016.

Chapter 2
Exploring Ethical Rationales

Lars Sandman, Kenneth Bond, and Björn Hofmann

2.1 Introduction

This chapter presents and analyses six ethical rationales for patient involvement in HTA. We have identified three instrumental and three substantive rationales, namely, (1) relevance to healthcare goals and healthcare needs, (2) legitimacy leading to adherence to decisions, (3) capacity building via patient empowerment, (4) fairness and legitimacy through democratic participation, (5) fairness through respect for autonomy and (6) equity. Our ethical analysis finds that these rationales support patient involvement in HTA under specific premises. For example, relevance to healthcare goals and needs mainly support the use of patient-based evidence, while the other rationales require patient participation in some form. That is, for HTA to be legitimate enough to increase adherence, patients probably need to participate in the process and the same goes for equity democratic participation, empowerment and autonomy. Importantly, in order to achieve strong ethical support for patient involvement in HTA, it is crucial to ensure that these premises and their preconditions are fulfilled. On the other hand, all rationales raise issues of representation, i.e. which patient group should be represented through evidence or participation.

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As HTA seeks to inform complex decisions in healthcare, it has always had an ethical underpinning, and, during the last few years, the work on ethics in HTA has undergone important developments (Hofmann 2008; Saarni et al. 2008). Patient involvement in HTA needs to be considered in this light. Ethical rationales may give defensible and well-supported answers to the question 'Should we do this?' and, if so, what are the ethical arguments for doing (or not doing) it. Ethical implications answer not only questions like 'What will happen (in terms of ethical values and norms) if we do this?' but also questions like 'How should we do this (in order to fulfil ethical values and norms)?' We provide an analysis touching on all these questions but focusing on whether or not it should be done. The aim of our critical analysis of the ethical rationales for patient involvement in HTA is to help those involved in HTA understand the premises for the rationales and thereby for the rationales to obtain and to ensure that patient involvement makes HTA better and provides better implications for the patients.

2.2 Ethical Rationales for Patient Involvement in HTA

Many rationales for patient involvement have been suggested (Gauvin et al. 2010; Kreis and Schmidt 2013; OHTAC Subcommittee 2015). We have chosen to follow the HTAi values for patient involvement in HTA of relevance, fairness, equity, legitimacy and capacity building (Chap. 1). Relating this list to central ethical aspects of healthcare, we get the following preliminary analysis. To assess relevance, we need to know to what the provided input should be relevant. The most obvious candidate in this context is the goal of healthcare, i.e. health and thereby healthcare need (Coulter 2004; Gauvin et al. 2010). To assess fairness, i.e. the right to participate, we need to acknowledge that such a right can be grounded both in an idea about democratic participation but also in an idea about respect for autonomy (Gauvin et al. 2010 Chap. 5). From an equity perspective, relevant information about healthcare needs is essential (Gauvin et al. 2010) but requires balancing different patient and other stakeholder needs. Legitimacy has a strong link to democratic participation, but while fairness emphasises the right to take part in decision-making (regardless of effects), legitimacy focuses on beneficial outcomes of participation in decision-making (Gauvin et al. 2010). Legitimacy is also related to the rationale of 'secur[ing] buy-in for contentious decisions' and thereby adherence to these decisions (Gauvin et al. 2010 [152]; (Bridges and Jones 2007). Finally, to assess capacity building, we need to decide exactly what patients should be given the capacity to do. However, besides this meta-role, involving patients could serve to empower the patients or patient groups, and empowerment (and its relation to autonomy and power) could be viewed as an ethical value in its own right.

The above rationales broadly fall into two categories: instrumental rationales and substantive rationales (Table 2.1). The instrumental rationales support patient involvement to the extent that involvement will have the 'right' beneficial effects.

Table 2.1 Ethical rationales for patient involvement in HTA

Instrumental rationales	Substantive rationales
Relevance to healthcare goals and healthcare needs	*Fairness* and *legitimacy* in terms of and through democratic participation
Legitimacy by leading to adherence to decisions	*Fairness* in terms of respect for autonomy
Capacity building by patient empowerment	*Equity*

The substantive rationales refer to ethical principles or norms supporting patient involvement, regardless of whether patient involvement will have any further beneficial effects or not. These substantive reasons do not exclude consideration of their effects, as we are interested also in knowing whether patient involvement in HTA will actually realise the norms or values behind these substantive rationales.

2.2.1 Different Roles for Patients in HTA

We analyse ethical rationales using the distinction made in Chap. 4 between patient-based evidence and participation. Following this, patient-based evidence refers to gathering data on patients' perspective using systematic research, and participation refers to being part of or provide individual input into an assessment or decision-making body within the HTA process.

2.2.2 Normative Analysis Methodology

An ethical rationale should be consistent with or result from established ethical principles, theories or perspectives, in contrast to just being an opinion or preference. To assess whether this is the case, we need to understand the meaning of the proposed ethical rationale, requiring conceptual analysis, and its implications for other values and norms. Hence, we analyse the rationales in relation to commonly accepted ethical values and norms of western healthcare using established conceptual understandings (Saarni et al. 2008).

2.3 Normative Analysis of Instrumental Rationales for Patient Involvement

This section is divided into analysing the instrumental and substantive rationales presented in Table 2.1.

2.3.1 Relevance to Healthcare Goals and Healthcare Needs

To promote the goal of healthcare, interventions must be effective and respond to relevant aspects of this goal. Health is the overall goal for most healthcare systems, and the most reasonable concepts of health relate to patients' subjective perspectives lending support for this rationale (Nordenfelt 2008). Hence, assessing whether there is a patient need of healthcare and the degree of this need involves knowledge about patients' subjective experiences (Gustavsson and Sandman 2014). Moreover, even the objective aspects of health cannot be achieved without knowledge about the conditions of the delivery of healthcare. A strong reason for patient involvement in HTA is therefore to adapt the assessment to relevant outcome measures (i.e. aspects of health) and take into consideration relevant issues affecting use at points of delivery (Coulter 2004; Gauvin et al. 2010; IAP2 2015). Still, we need to address some critical aspects in regard to how patients' subjective experiences and perspectives should be taken into account.

First, there is disagreement about the extent to which patients' subjective perspectives should be taken into account (Nordenfelt 2008). There is no known account of health where it is simply up to individual preferences to define what health is, and normally one distinguishes between patient need and preferences, emphasising the importance of the former (Gustavsson and Sandman 2014). Hence, there needs to be a general discussion about how patients' subjective perspectives should be balanced against other aspects that should be taken into account.

Secondly, we need to deal with the possible diversity of subjective perspectives among patients. Within the healthcare sector, we see a strong trend towards person-centred care, based on the reasonable and empirically supported assumption that 'one size does not fit all' (Munthe et al. 2012). Hence, we need to decide how to deal with the possible diversity of how to adapt outcome measures, what aspects of delivery to take into account, etc. This might be called *the selection problem.* How do we ensure that all the different patient perspectives on health are taken into account? Should we take all perspectives into account, regardless of frequency in the patient population, or should we focus on a few common perspectives? Moreover, how do we balance the different patients' or patient groups' perspectives on health if they favour conflicting outcomes or modes of delivery. This implies that involving patients is likely to make the assessment *more* relevant to certain patients, or patient groups, but this might not increase its relevance for other patients or patient groups (and might even decrease relevance for them). Achieving relevance per se is therefore likely to be an impossible ideal to achieve, and we need to be aware that relevance is likely to come in degrees.

Thirdly, we need to consider *the effectiveness problem.* Making HTA more relevant to patients might increase the complexity of the assessment as a result of being aware of issues for which there is no strong data or conflicting data. Hence, relevance might come at the expense of effectiveness of HTA, if effectiveness is measured in terms of HTA resulting in strong support for clear-cut recommendations. This might also be a challenge in relation to public health policy if patient

involvement in HTA results in outcomes that are contrary to public health goals, since these tend to be less adapted to subjective perspectives.

Generally, the relevance rationale is best served through patient-based evidence and systematic qualitative and quantitative research of patient perspectives and views (rather than patient participation). However, even in research there might be a *problem of representation* (i.e. do the research participants actually represent the diversity of the patient group in question—Chap. 3).

2.3.2 Legitimacy by Leading to Adherence to Decisions

If a technology is assessed using patient-relevant outcome measures and adapted to patient needs, it will be more likely to be used by the patients. (Gauvin et al. 2010; Bridges and Jones 2007). Given this, the selection and effectiveness problem will affect this rationale. That is, decisions are likely to be accepted primarily by those patients or patient groups for which they are found to be relevant, and it might be more difficult to achieve adherence if the decisions are less clear-cut. A further possible problem is that if the HTA is made more relevant for patients, some professionals might find it less relevant (according to professional standards), affecting professional adherence to decisions and use. Claiming that professionals should simply accept a patient's perspective on their own treatment is challenging, as patients' demands may not be ethically warranted. Accordingly, in the discussion on patient or person-centred care, it is questionable whether we should accept patients' perspectives without limitations, and it is argued that the most reasonable standpoint is to find a balance between patient and professional or systemic views on relevance (Sandman and Munthe 2009).

Another way to achieve adherence or acceptance of decisions is through an ambassadorial role for the involved patient representative. In distinction to the relevance rationale, an ambassadorial role calls for participation in the HTA process to get an understanding of the technology, its delivery and its assessment. In such a role, patient representatives could advocate for acceptance and adherence to both positive and negative recommendations. This rationale should be viewed as a supportive, rather than primary, argument for patient involvement as patients are unlikely to consider it a convincing argument for involvement on its own.

Several premises are crucial to the legitimacy rationale.

First, there is a challenge with representativeness. This is a general challenge for patient involvement and calls for an analysis of its own, i.e. given diversity of patients and patient experience and perspectives, how are these patients or perspectives best represented in HTA (Gagnon et al. 2015). In relation to the rationale at hand, the patient representative needs to have a strong and trusted standing in the patient community in order to act as an ambassador with actual impact on adherence and acceptance.

Secondly, the more patient representatives become involved in the HTA process, the more they can be viewed as professional patient representatives, perhaps even

more closely aligned with the HTA community or with other more influential groups than the patient group they represent (Chap. 3). Milewa (2008)) describes how some patient groups link with representatives from the pharmaceutical industry to further their own interests, something that might in the long run compromise their impartial standing in the patient community. To maintain a 'trusted voice' as patient, representatives might require constant awareness and support from both the patient group and the HTA community.

Thirdly, whether involving patients as ambassadors will actually result in better acceptance or adherence to HTA decisions or recommendations is an open question. Insight into and understanding of the HTA process and its outcome for a specific technology might even lessen support for the resulting decision. Using the patient representative to convince patient groups of the HTA community's views would run counter to values like respecting patient autonomy or empowering patients and can be manipulative and paternalistic. Accepting patient representatives as independent of the HTA community and as advocates of patient interests and perspectives might not always make them effective ambassadors for accepting or adhering to HTA decisions. This calls for a discussion on whether there are constraints on how the patient representative should be expected to act in an ambassadorial role.

2.3.3 Capacity Building by Empowerment

Ethicists have observed that the scope of autonomy in healthcare has broadened over the last few years from respecting patient autonomy to also strengthening autonomy (usually called empowerment) (Sjostrand et al. 2013). Accepting this as a relevant ethical norm for the healthcare sector, in general, would also lend support to the rationale of capacity building.

How can patient involvement empower patients or patient groups in the HTA process? Information is essential to exercising autonomy effectively, and, being involved in the HTA process, patients might be given better access to relevant information. Obviously, empowering patients in HTA requires that the participating patient representatives make their patient groups better informed and not only individual participating patients. Participants in the HTA process can gain an understanding of different aspects relevant to patient decision-making that would not be possible without such participation. In addition to communicating such information to their patient groups, they may also be able to convey information needs to HTA bodies. Hence, as there is some clear support for this rationale, we also examine a few possible challenges that may be faced in making the rationale sound.

First, if we interpret empowerment in terms of being in a better position to actually make and execute decisions, more information might not always make us more effective decision-makers. Instead, it might become stifling for the exercise of autonomy, for example, when it reveals a more complex situation and makes it difficult to choose between the alternatives. Such stifling information could include

limitations of data, methodological weaknesses, impact on healthcare budgets and issues for healthcare professionals. This is not an argument for the HTA community to keep patients in the dark, but rather an argument for patient representatives to judge the type and extent of information that can empower their community.

Secondly and related to the selection problem given diverse patient preferences and perspectives, different information might empower different patient groups. Patient groups not represented in the HTA process might not receive the relevant information.

Groups are also empowered by the opportunity to influence or make decisions in the HTA process. Here we find similar selection problems as before. By empowering one patient group, other groups affected will not be correspondingly empowered, and the interests of the empowered group might not be in the best interest of all patients or the society. Hence, strengthening a specific group may be counter to fairness.

The issue of group representation raises a more general issue: Is it possible to empower a *group* by allowing a *group representative* to take part in or make decisions? It is only plausible if the group has a set of consistent interests that can be represented in a set of decisions by the representative. Even if involvement of a representative does not lead to the empowerment of the whole group, it can lead to empowerment of a subcategory of this group, i.e. the ones whose preferences, values and expectations are represented in the decisions or the ones who can identify with this process.

These problems point to some preconditions and limitations of this rationale and the need to make value judgments for whom, and to what extent, empowerment is important and should be strived for.

2.3.4 Concluding Comment about Instrumental Rationales

It is in the nature of instrumental rationales that their success lies in that they will result in the intended consequences. This depends on a number of empirical factors. We have argued that even if the valuable ends in question have strong support to be achieved, they do not *necessarily* follow from patient involvement in HTA or at least that there are several premises for when the ends will be obtained. Hence, these premises need careful attention. To find even stronger support, we should turn to the substantive ethical rationales for patient involvement (Entwistle and Watt 2013).

2.4 Normative Analysis of Substantive Ethical Rationales for Patient Involvement

Regardless of any positive effects of patient involvement in HTA, it can be argued that we have substantive ethical reasons for involving patients.

2.4.1 *Fairness and Legitimacy Through Democratic Participation*

A basic feature of most democratic theories is that all stakeholders concerned should be allowed to have a say in matters pertaining to them (Gauvin et al. 2010; Facey et al. 2010). In our opinion, the strongest support for involvement can be found in ideas about deliberative democracy (Chap. 13). The idea that it is an ideal for democratic governance that citizens provide informed and deliberative input has been advocated as support for involving patient representatives in decisions concerning ethical conflicts in biomedical research (Kim et al. 2009). In this discussion, patient involvement is contrasted with the idea of patient advocacy groups or representatives. Specific interests drive the latter, while the former involvement is based on an idea about the common good. Hence, from a fairness perspective, the idea about deliberative democratic participation seems more fruitful. At the same time, patient involvement might need to be complemented by representatives of the general public, i.e. the taxpayers in a welfare society, or other patient groups that might be indirectly affected by HTA decisions.

The basic goal of deliberative democracy is to reach a policy recommendation. Hence, such a rationale might above all support involvement in strategic policy decisions, for example, on coverage policies, guiding values of the HTA process, decisions about controversial technologies, etc. This requires that the different representatives of the relevant stakeholders are willing to accommodate each other's perspectives and arguments to reach consensus and not through majority rule (Fishkin and Luskin 2005). Deliberative democracy has a clear affinity with discourse ethics, with ideas about free expression of arguments, openness to change, a willingness to take other parties' perspectives and not allowing power relationships to influence the deliberation (Habermas 1984). We point to a few possible challenges given this rationale.

First, even when accepting these constraints, not all decisions resulting from a deliberation are acceptable and can be a matter of democratic decision-making. If we accept some central values in the healthcare system, not all value judgments will be supported if we want to be consistent and maintain the possibility of rational argument. For example, even if a majority of the population find it warranted to discriminate against certain groups when it comes to healthcare treatment, this is not acceptable given general values and norms about equality and fairness. Still, if rational argumentation does not give a definite answer, democratic decision-making might be our best option to resolve the issue.

Secondly, even if we identify matters suitable for democratic decision-making, we are still left with the question of proper representation. Thirdly, there are difficulties of avoiding power relationships influencing the decision given group dynamics (Ryfe 2005) and the problem of reaching a common decision (Sunstein 2007).

2.4.2 *Fairness Through Respect for Autonomy*

A dominating norm within western healthcare is respect for patient autonomy that the decision of a competent person should be respected. Such respect requires that the patient is allowed to *make* the decision, i.e. requires patient participation in decision-making within HTA (Sandman and Munthe 2009).

Can we respect the autonomy of a group by allowing a representative of this group to make a decision? In other contexts, it has been argued that respecting the decision of a representative of a patient does not imply that we have respected the autonomy of the patient, since the patient did not make the decision (Brostrom et al. 2007). This would be so even if he or she makes the same decision as the patient would have made on the exact same grounds.

According to a less strict interpretation, the autonomy of a patient group is respected if a legitimate representative makes a decision based on the group's preferences and interests (and these are aligned within the group). This is definitely a relevant rationale, even if it might need further theoretical development on how to understand group autonomy. Nonetheless, we can point to a few possible questions that need answering.

First is the level of understanding of patient representatives sufficient to be autonomous in the HTA process? Since understanding is a matter of degree, different patient representatives can be more or less autonomous in the process. (This of course is also relevant for other decision-makers.)

Secondly, to decide autonomously a person needs enough decision competence (Sjostrand and Juth 2014). Some patients, especially those with different cognitive abilities, might not be able to reach this level of competence and would have difficulty finding a representative *from* their group (even if they might find representatives *of* their group).

Thirdly, even if decision competent, training and education could still raise autonomous ability. A possible paradox in training patient representatives to be knowledgeable in HTA is that this might affect their attitudes or preferences concerning the HTA process, turning them into professional patient representatives or even part of the HTA establishment.

Yet another challenge is voluntariness, as patient representatives may have strong directives from their organisations that conflict with their own conceptions.

Different approaches may be necessary in order to satisfy the premises of the rationale of respecting the patient group's autonomy.

2.4.3 *Equity*

In many countries, HTA is an important part of the process of priority setting in healthcare. It is, however, not a sufficient input, since the output of the HTA process is often complemented by considerations of patient needs and cost-effectiveness in

the priority setting process (Hofmann 2013). Even if these values are relatively well determined (e.g. countries like Sweden and Norway have explicit sets of principles and criteria for how to prioritise in healthcare), the process of applying these values is open to influence from patient groups.

Which technologies are under consideration for funding? How are the values interpreted in relation to specific technologies? How are aspects like patient needs assessed? These questions point to areas where participants in HTA and priority setting processes can affect the outcomes. To allow patients, especially disadvantaged patient groups, to have a say in these matters could result in fairer access to treatment. Providing input through patient-based evidence could go part of the way towards such a goal, but real influence over distribution obviously calls for participation in deciding on coverage policies or actual coverage. Again, it is wise to consider a few possible challenges with this rationale.

First, we have a version of the *selection problem*; in allowing one specific patient group to be represented, there is a risk of unfair distribution as the group advocates for their own interests at the expense of other stakeholders. If, on the other hand, we involve a more general representative of different patient groups or the public, this might risk reproducing existing prejudices within the population (that might be at odds with codified values concerning fairness).

Secondly, if patient representatives manage to influence decisions in a way that is unsupported by reasonable interpretations of the evidence, simply because this is something in demand among their patient peers, it would seem problematic from a need-driven account of fairness. This is something to be especially wary of when it comes to patient advocacy groups with influential leaders or when patient groups and representatives are strongly influenced by or financially supported by the pharmaceutical industry (Milewa 2008). So, the problem of representativeness is evident.

Accordingly, equity is a relevant rationale for patient involvement, but to make it sound, we need to address its premises. Generally, as equity issues are about how different parts of a system are related to each other, it calls for a systems approach to some extent and disregarding participants' idiosyncratic perspectives. It could be argued that groups having a very strong stake in the decisions, for example, patients, are not in the position to take on such a systems perspective and should not be expected to. Accepting this view on equity would lend support for patient-based evidence rather than participation, in order to assess aspects like patient need, relevant effects on patients, and relevant costs.

2.5 Some Final Thoughts

In this chapter, we have provided a normative analysis of some of the strongest ethical rationales for involving patients in HTA. While patient involvement is clearly supported by a wide range of ethical rationales, there are also preconditions for these rationales to be sound. Our critical analysis is not motivated by scepticism about the possibility of justifying patient involvement. On the contrary, by highlighting important premises and their corresponding challenges,

we have pointed to issues that HTA bodies need to address in order for the rationales to apply and to realise ethically well-justified patient involvement as described in Table 2.2.

Table 2.2 Overview of ethical rationales, types of involvement and questions to consider

Rationale	Patient involvement	Questions to consider in HTA
Relevance to healthcare goals and needs	Patient-based evidence	• How should patients' subjective perspectives be balanced against more objective aspects of the goal of health or healthcare need in general? • How to handle the selection problem of diversity of patient perspectives? • How to handle the effectiveness problem if ending up with less clear-cut HTA recommendations?
Legitimacy by leading to adherence to decisions	Patient-based evidence (for the relevance aspect) and patient participation (for the ambassadorial role)	• Consider the above questions about relevance • How to handle possible conflicts between patients and professionals concerning relevance affecting adherence and acceptance? • Who has the proper standing to act as ambassador? • How should we minimise the risks of ambassadors becoming part of or hostage to other interests than patient interests? • How should we prioritise between achieving adherence and acceptance and allowing the patient representative to be an independent representative of his/her patient group (regardless of effects on adherence/acceptance)?
Capacity building by empowerment	Patient participation	• How to balance between information distribution and effective decision-making of the patient group when they are in conflict? • How to handle the selection problem when distributing information? • How to handle the selection problem when involving patient representatives in actual decision-making? • How to handle possibly resulting fairness or equity problems resulting from the selection problem?
Fairness and legitimacy through democratic participation	Patient participation	• How should we solve the problem of representativeness in relation to the concerned stakeholder of HTA? • How should we delimit the aspects of the HTA process that are not negotiable through democratic decision-making? • How should a democratic process be organised to avoid undue power influences from certain groups? • How should a democratic process be organised to achieve joint and common, yet still effective, decision-making?

(continued)

Table 2.2 (continued)

Rationale	Patient involvement	Questions to consider in HTA
Fairness through respect of autonomy	Patient participation	• How to handle the selection problem when acting as a decision-maker on behalf of a patient group? • How to handle possible fairness or equity problems resulting from the selection problem? • How to ensure that patient representatives are knowledgeable enough to be considered autonomous? • How should patient groups, generally lacking autonomous ability due to cognitive problems, be represented? • How to empower patient representatives to become more autonomous and still remain representatives of the patient group?
Equity	Patient-based evidence or patient participation	• How to avoid equity problems arising from the selection problem? • To what extent should patient perspectives be allowed to influence more general norms about what constitutes an equitable distribution?

References

Bridges JFP, Jones C. Patient-based health technology assessment: a vision of the future. Int J Technol Assess Health Care. 2007;23:30–5.

Brostrom L, Johansson M, Nielsen MK. "What the patient would have decided": a fundamental problem with the substituted judgment standard. Med Health Care Philos. 2007;10:265–78. doi:10.1007/s11019-006-9042-2.

Coulter A. Perspectives on health technology assessment: response from the patient's perspective. Int J Technol Assess Health Care. 2004;20:92–6.

Entwistle VA, Watt IS. Treating patients as persons: a capabilities approach to support delivery of person-centered care. Am J Bioeth. 2013;13:29–39. doi:10.1080/15265161.2013.802060.

Facey K, Boivin A, Gracia J, Hansen HP, Lo Scalzo A, Mossman J, et al. Patients' perspectives in health technology assessment: a route to robust evidence and fair deliberation. Int J Technol Assess Health Care. 2010;26:334–40. doi:10.1017/s0266462310000395.

Fishkin JS, Luskin RC. Experimenting with a democratic ideal: deliberative polling and public opinion. Acta Polit. 2005;40:284–98. doi:10.1057/palgrave.ap.5500121.

Gagnon MP, Desmartis M, Gagnon J, St-Pierre M, Rhainds M, Coulombe M, et al. Framework for user involvement in health technology assessment at the locale level: views of health managers, user representatives and clinicians. Int J Technol Assess Health Care. 2015;31:68–77. doi:10.1017/s0266462315000070.

Gauvin F-P, Abelson J, Giacomini M, Eyles J, Lavis JN. "it all depends": conceptualizing public involvement in the context of health technology assessment agencies. Soc Sci Med. 2010;70:1518–26. doi:10.1016/j.socscimed.2010.01.036.

Gustavsson E, Sandman L. Health-care needs and shared decision-making in priority setting. Med Health Care Philos. 2014;18(1):13–22.

Habermas J. The theory of communicative action. Boston: Beacon Press; 1984.

Hofmann B. Priority setting in health care: trends and models from Scandinavian experiences. Med Health Care Philos. 2013;16:349–56. doi:10.1007/s11019-012-9414-8.

Hofmann BM. Why ethics should be part of health technology assessment. Int J Technol Assess Health Care. 2008;24:423–9. doi:10.1017/s0266462308080550.

IAP2. Public participation spectrum. 2015. https://www.iap2.org.au/documents/item/83.

Kim SYH, Wall IF, Stanczyk A, De Vries R. Assessing the public's views in research ethics controversies: deliberative democracy and bioethics as natural allies. J Empir Res Hum Res Ethics. 2009;4:3–16. doi:10.1525/jer.2009.4.4.3.

Kreis J, Schmidt H. Public engagement in health technology assessment and coverage decisions: a study of experiences in France, Germany, and the United Kingdom. J Health Polit Policy Law. 2013;38:89–122. doi:10.1215/03616878-1898812.

Milewa T. Representation and legitimacy in health policy formulation at a national level: perspectives from a study of health technology eligibility procedures in the United Kingdom. Health Policy. 2008;85:356–62. doi:10.1016/j.healthpol.2007.09.001.

Munthe C, Sandman L, Cutas D. Person centred care and shared decision making: implications for ethics, public health and research. Health Care Anal. 2012;20:231–49. doi:10.1007/s10728-011-0183-y.

Nordenfelt L. The concept of work ability. P.I.E. Peter Lang: Bruxelles, New York; 2008.

OHTAC Public Engagement Subcommittee. Public engagement for health technology assessment at health quality Ontario–final report from the Ontario health technology advisory committee public subcommittee [internet]. Toronto: Queen's Printer for Ontario; 2015. http://www.hqontario.ca/Portals/0/documents/evidence/special-reports/report-subcommittee-20150407-en.pdf Accessed 27 Aug 2016

Ryfe DM. Does deliberative democracy work? Ann Rev Pol Sci. 2005;8:49–71.

Saarni S, Autti-Rämö I, Lühman D, Hofmann B, Velasco-Garrido M, Lopez de Argumedo M et al. Ethical analysis. HTA core model for medical and surgical interventions 2008. https://meka.thl.fi/htacore/model/HTA%20Core%20Model%20for%20Medical%20and%20Surgical%20Interventions%201.0r.pdf

Sandman L, Munthe C. Shared decision-making and patient autonomy. Theor Med Bioeth. 2009;30:289–310. doi:10.1007/s11017-009-9114-4.

Sjostrand M, Eriksson S, Juth N, Helgesson G. Paternalism in the name of autonomy. J Med Philos. 2013;38:710–24. doi:10.1093/jmp/jht049.

Sjostrand M, Juth N. Authenticity and psychiatric disorder: does autonomy of personal preferences matter? Med Health Care Philos. 2014;17:115–22. doi:10.1007/s11019-013-9509-x.

Sunstein CR. Group polarization and 12 angry men. Negot J. 2007;23:443–7. doi:10.1111/j.1571-9979.2007.00155.x.

Chapter 3
Reflections on Terms, Goals and Organisation

Helle Ploug Hansen and Jackie Street

3.1 Introduction

In this chapter, we address three challenges relating to patient involvement in HTA. Firstly, we reflect on some of the terms often used by HTA researchers, patient organisations and HTA bodies to describe the patients who participate in HTAs including terms such as 'patient', 'patient advocate', 'patient representative', 'patient partner' and 'consumer'. This challenge has previously been described in relation to healthcare in general. Dent and Pahor write: 'The whole arena of patient involvement within healthcare is riven with problems of meaning, definition and purpose' (Dent and Pahor 2015, p. 549). In addition, they argue that the topic is further complicated when one attempts to compare practices across countries (Dent and Pahor 2015). Secondly, we discuss how the choice of term and hence the choice of participants may influence the realisation of goals with patient involvement in HTA. We argue that a challenge lies in confusion about the goals for implementing patient involvement in HTA, particularly when these goals compete with a variety of other goals such as cost containment or decision-making based on strictly defined clinical effectiveness. Thirdly, we address the challenge relating to leadership and organisational change, because some of the goals for patient involvement in HTA will require new ways of organizing and leading HTAs. We briefly introduce three models for organising patient involvement in HTA. The aim of the chapter is to contribute to the current debate on the use of terms to describe patient involvement and the nature of the associated goals and organisation supporting this involvement.

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Traditionally, in some countries, HTA has been seen as the provision of independent evidence-based assessment of the safety, clinical and cost-effectiveness of health technologies with patients' experiences and preferences as useful but not essential components (Chap. 1). In other countries such as Denmark, patients' perspectives and experiences have been an integrated part of a full HTA (Kristensen and Sigmund 2007). Part III of this book is a presentation of how different countries around the world have worked with patient involvement in relation to HTA. However, although EUnetHTA (Chap. 24) has integrated patient and social aspects in their HTA Core Model® and presents these aspects as an integrated part of an HTA process equally with other domains, EUnetHTA states that economic evaluation and clinical effectiveness until now have played a much more dominant role in HTAs.

In addition, some HTA researchers and decision-makers receiving HTA reports see the introduction of patient involvement in HTA as a potential hazard in achieving independent evidence-based decisions and question the ability for 'nonexpert' or 'lay' members of the public to be involved in highly technical assessments and decisions (Russell and Greenhalgh 2014; Lopes et al. 2015). On the contrary, others believe that we have only begun to incorporate patients' perspectives and involve them in the HTAs to incorporate patient values and need to further develop patient involvement (see Sect. 3.3). To meet this challenge, it will be necessary for HTA practitioners to acquire new skills, new knowledge, new practices and new competencies. Similarly, shared understandings of the relationships between HTA researchers, practitioners and patients will need to be developed, critically reflected upon and implemented. Reflection about leadership and distribution of power in decision-making are therefore crucial to the HTA process.

Overall, there may be a tension between the evidence arising from clinical trials and economic models and the patient-based evidence arising from the perspectives and experiences of patients (Chap. 4). In particular, the value placed on a service or technology by patients may run counter to the values expounded in assessments. Patient values may be different to those held by other experts in the HTA process or by citizen at large (Street et al. 2008). In addition, patients' views and preferences, similar to the views and preferences of health technology developers and clinicians, may be skewed by a vested interest in the outcome. These interests need to be acknowledged and managed appropriately. HTAs need to be patient centred (Bridges and Jones 2007) but must also recognise what is best for all patients across the board (see evaluation of equity in Chap. 2). It is possible that since patient groups often value a fair and transparent process, as long as these two conditions are met, they may be reconciled to the outcome. However, this notion itself deserves further scrutiny.

Understanding who will be asked to contribute, how and when, is important in sorting out this potential conflict of views in HTA. We argue that reflection on the terms used to describe the patients that are involved in HTA and the ideas underpinning their use is essential before we can discuss and consider how, when and on what basis patient involvement can be effectively implemented in HTAs. In particular, we argue that we need deep reflection on the overarching goals of patient involvement in HTA and the role of patients and their representatives in the HTA processes.

3.2 Terms Used to Describe 'Patients' in HTA

The terms used in HTAs to describe patient involvement are crucial in defining the roles and positions of those involved. A review of the role of patient and public participation in HTA, published by Menon and Stafinski (2011), provides an indication of the range of the terms used to describe such participants. We argue that the choice of term to some degree defines the type of participants who will be involved, their specific qualifications and competences and their interests. Work to define the goals for public and patient participation and hence the nature of the selected participants is already well underway in some organisations. For example, the European Medicines Agency (EMA) only invites patients to sit on scientific advice committees discussing a clinical trial, in order that the committee may hear the experience of someone living with the disease (EMA 2014). However, only European umbrella organisations may sit on committees that shape the processes and policies in order to incorporate the broader policy perspectives of the organisations. Nevertheless, in reading HTA reports and HTA articles, it is clear that, in many cases, the terms chosen are used with little reflection or consideration of these issues. In the following, we reflect on some of the terms often used to describe patient involvement in HTA.

3.2.1 Patient

Etymologically the concept 'patient' stems from Latin *patientem*, meaning bearing, supporting, suffering, enduring and permitting (Harper 2016). Today the term 'patient' usually refers to an individual with a diagnosed disease or disorder who is using or has used the healthcare system due to their need for treatment and care. In HTA this term is often used in a general way to state that patients' views, experiences, preferences, needs and involvements are of importance although frequently without reference to why that is the case. The homepage of the HTAi Interest Group for Patient and Citizen Involvement in HTA (the HTAi Interest Group) states, for example, 'Our vision: Patient and citizen perspectives improve HTA' (HTAi 2016a). Throughout the different links on the website, the term patient is taken for granted in that no explicit reflections on the nature of the term are presented in the text. The most detailed exploration of the term can be found in the stated aim of patient involvement: 'to ensure that HTA assessments and decisions are informed by the special knowledge/unique perspectives of those with the lived experience of a health condition and its management, or who are able to speak on behalf of patients as their informal carers' (HTAi 2016a).

Some authors explicitly describe the term *patient.* Bridges and Jones (2007, p. 32) do so by describing what a patient is not: 'To be relevant, the patient's perspective (rather than one of a citizen, tax payer, provider, or payers) must be addressed'. In contrast, Facey et al. (2010, p. 335) describe a patient as 'any current or potential health service user or beneficiary of a health technology' and 'a person who has

valuable experiential knowledge about a specific illness or condition…'. The inclusion of a broad spectrum of patients in HTAs is difficult. For example, it is likely that patients with no or low education, homeless patients, patients living alone, patients experiencing high levels of 'social shame' (such as with venereal diseases, alcohol dependence or substance misuse) or patients of lower social position may be more difficult to include in an HTA. Although many organisations are conscious of these omissions and seek to be inclusive, we would argue that patient representation in an HTA will always be incomplete and the notion of inclusion of 'patient views' inherently flawed. See Sect. 12.8 for the production of knowledge, where the authors state that knowledge is always both reductive and selective.

3.2.2 Patient Advocate and Patient Representative

The terms patient representative and patient advocate are often used interchangeably. This is, for instance, the case on the homepage of the HTAi Interest Group in the Frequently Asked Questions (HTAi 2016b). Here, a distinction is made between a patient and a patient representative or advocate in HTA, but not between an advocate and a representative. Hofmann and Saarni (2011) see advocacy as a potential aim for the patient representative in supporting 'empowerment' and 'autonomy' for patients and 'improvement of the health systems' Alternately the role may be seen as more circumscribed demanding 'more and better services' for their patient group (Hofmann and Saarni 2011). In a qualitative study from Australia, some participants 'questioned the legitimacy of umbrella organisations and some patient ("consumer") representatives in representing the wide range of patients and patient organisations with some interest in the process (Lopes et al. 2015). In this case "patient advocate" appears to be a more appropriate term since the individual will advocate on behalf of patients generally but does not necessarily "represent" all patient groups. Patient advocates or representatives are described as participating on behalf of patients or representing the views of a particular group of patients, survivors or carers: "Sometimes this will be for people who have a debilitating or rapidly progressing disease or limitations in their communication skills"' (HTAi 2016b). The term patient advocate or representative may also be used to describe a professional who supports the patient and their caregivers (carers) through the illness journey and assists them in negotiating with the often complex health systems. In HTAs these terms are usually used to describe an individual or an organization promoting the interests of a broad group, for example, cancer survivors or patients with a rare disease or the views of a very specific group, such as patients with Alzheimer's disease. Patient advocacy organisations are frequently non-profit. The modern concept of the patient advocate arose in the 1950s with concerns about the involvement of cancer patients in research trials (Keating and Cambrosio 2012).

If a health technology is contentious with entrenched views held in different patient advocacy organisations, there is potential for the debate to become polarized. In this case, the views of a vocal minority—not necessarily shared by the broader

patient community—may skew the HTA processes. For example, the broader views of the deaf community may not have been heard in the debate as to whether prelingual deaf children should receive cochlear implants (see Batterbury 2008). However, there are few published examples of this phenomenon, and therefore, it is difficult to judge the real impact in HTA. Alternatively, the interests of high profile diseases, such as breast cancer, with numerous strong patient advocacy organisations, may dominate, such that those advocating for low-profile diseases struggle to be heard. In aiming for a balanced deliberation and community discussion about the adoption and/or public funding for a particular technology, patient advocates may be bound by the community they serve and may not be able to respond reflectively to evidence which runs counter to the community view. Similarly, some HTA practitioners have expressed concern about the influence of the pharmaceutical or medical device industries on the views propagated by patient advocacy organisations (Lopes et al. 2015). Caregivers may also act in the capacity of patient advocate or representative. This may be a challenge, since caregivers 'may have their own experiences and views which can form part of the knowledge base' (HTAi 2016b). However, it is important to stress that the needs of the caregivers are important and that they need to be considered as representatives in their own right.

3.2.3 Patients as Consumers

Some researchers have argued that the term consumer is broad enough to encompass patients, members of the general public, caregivers, etc. (Royle and Oliver 2004; Bastian 1998). Certainly, the term includes citizens who irregularly access systems of healthcare, for example, for screening programs or vaccines, but who may not be seen as 'patients'. However, including patients within this umbrella term assumes an equal relationship between the 'seller' and the 'consumer'. In the case of a patient, such a relationship would generally not be possible since patients frequently have few options in terms of their treatment and insufficient information on which to make choices and decisions. In addition, the patient may be seriously ill and burdened with the demands of his/her disease. Some diseases lend themselves more readily to the notion of patients as consumers than others, that is, the disease burden may not be as high, there may be a large number of long-term survivors or the disease may be better understood in the general population. In general, although patients are consumers of health technologies, the usual contractual arrangement between seller and buyer does not apply since it is clearly an unequal partnership. In most cases, the patient, particularly a patient in a publicly funded system, is unable to change their provider or treatment options or it may be difficult to do so. Tritter (2009, p. 285) states that it is important to be aware of the tensions between involvement and consumerism, since promoting patients as consumers may limit the evolution of patient and public involvement. The term might be considered particularly inappropriate in the case of shared decision-making which is based on mutual respect rather than a commercial agreement.

3.2.4 Patients as the Public

Because of their potential for partisan views (3.3), viewing the patient as representative of the broader public is usually inappropriate. On the other hand, the assumption that patients will not recognize the broader societal consequences of particular decisions may also be erroneous (Hodgetts et al. 2014). Clarity around the role of public or patient input will assist in teasing the two areas apart. That is, in HTAs, the public interest and the patient interest are usually different, and therefore, their input should be collected separately. Individuals who represent the interests of patients should not also be required to represent the interests of the broader public. In an article which conceptualised the term public involvement, Gauvin et al. (2010) highlighted some of the challenges: for example, their research indicated that there was little agreement in the literature as to who 'the public' is or ought to be, or the most appropriate terms to define 'the public' (Gauvin et al. 2010, p. 1522). They argued that until recently, the public was referred to as 'consumers' as a reflection of the market-oriented ideologies of the 1970s and 1980s.

3.2.5 Patients as Experts

Patients may be considered experts of their own experience of the disease condition and in terms of the applicability and importance of any technological application. This term is often connected to the argument of why to involve patients in HTA. For instance, Geissler from the European Patients' Academy (EUPATI) wrote: 'They [patients] are the experts on quality of life. They know how it is to live with a disease. They can probably assess one against the other…' (EUPATI 2016). 'Informed research, assessment and decision-making are not possible unless patients are involved…'. The term patients as experts is diametrically opposite to the idea of patients as 'lay' representatives. A patient as expert may be defined as a patient representative of a specific group of patients. They may also be involved as experts together with different experts among the healthcare professionals, because of their knowledge, networks and ability to contribute. Today different organisations such as EUPATI offer training courses for patients as experts. In an article by Hartzler and Pratt (2011), the authors discuss the different input that patient's expertise and clinicians' expertise may contribute. For example, they demonstrate that where patient expertise contained personal topics carried through narrative-style action strategies and perspectives, clinician expertise was medical, knowledge oriented and prescriptive (Hartzler and Pratt 2011).

The different terms we have reflected on here are all normative statements. Normative statements are usually understood as positive or negative, good or bad or right or wrong. In relation to research or HTAs, the terms such as patients as experts, patient representative and patient advocate or patients as partners (Chap. 8) are valued as positive and good. Positive statements are often taken for granted becoming factual statements attempting to describe reality. Although normative statements and norms and their meanings are an integral part of human life, it is important that

HTA researchers reflect on them and, for instance, ask questions such as 'What types and kinds of meaning are inherent in the terms we use'.

3.3 Goals for Patient Involvement in HTA

The terms the HTA researchers choose to use and hence the choice of participants may influence the realization of goals with patient involvement in HTA including capacity building, patient empowerment and the knowledge gained. Although it is not always apparent why HTA organisations involve patients in HTA, there are a range of potential goals for patient involvement (Abelson et al. 2007). The choice of goals sets in motion a 'different set of instruments and actors' (Abelson et al. 2007, p. 40). Defining the goals for patient involvement will help define the type of participants needed to meet these goals. In HTA these goals may be:

- *Democratic goals* seek to achieve more informed, transparent, accountable and legitimate assessment to improve the decision-making processes. As such the processes must be seen as fair and transparent drawing broad-based support from patients and the broader community. Transparent representation of stakeholder views safeguards against perceived bias in HTA and in particular bias due to political pressure in decision-making (Busse et al. 2002; Liberati et al. 1997; Gallego et al. 2011; Van der Weyden and Armstrong 2004). Furthermore, these goals draw on the notion that citizens have rights and responsibilities which extend to patients as citizens. Including citizens in decisions which will directly affect them is a fundamental tenet of participatory or deliberative democracy. Patients in HTAs are the citizens who will be most impacted by an assessment report and the ensuing decision, and therefore, it could be argued and have a special place in the HTA process
- *Scientific goals* seek to promote a more robust and comprehensive scientific basis for HTA that incorporates social and ethical values (Chap. 2), as well as patients' issues, lived experiences, outcomes and preferences
- *Instrumental goals* are based on the belief that involving patients in HTA will improve the HTA assessment and make better quality decisions across all stages of an HTA. Understanding the lived experience with a disease may be crucial to the way in which an assessment is framed and conducted. For example, Street et al. (2008) and Facey et al. (2001) drawing on reports of patient experience demonstrate how the lived experience with diabetes may impact on the acceptability and uptake of technologies to detect and treat diabetic retinopathy
- *Developmental goals* are tied to the move towards increased patient empowerment with patients as partners in their own care. Not involving patients in HTAs restricts the ability of patients to define the treatment choices available to them, a fundamentally disempowering and disenfranchising move. Increasing public understanding of health technologies and HTA strengthens the public's and patients' competence and capacity to contribute to health technology policy issues (Gauvin et al. 2015)

On occasion, depending on how and when patients are involved in the HTA process, these goals may conflict or give rise to bias or skewed assessment particularly since patients and caregivers involved in an HTA may have a vested interest in the outcome. This may be particularly the case where an expensive medicine only extends life by a few weeks or months or does not show long-term benefit. For this reason, patients, patient advocates or patient representatives might in some cases be viewed as 'partisans' in that they may have strongly held beliefs about a technology and therefore less amenable to engagement in 'evidence-based' discussion. In particular, a group of patients may be influenced by a persuasive individual or through the input of stakeholders such as clinicians or health technology developers. This does not necessarily mean that their views are unreasonable or that they should not be included in debate but rather that those patient groups may be unwilling to change their views in the light of clinical evidence. In mitigation, we should also consider that evidence-based medicine itself represents a particular world view and in some cases may neglect the needs and priorities of patients (Jensen 2004; Hansen 2004) and that many systems have processes for management of such conflicts of interest. Yet in some cases, the impact of partisan views may result in coverage for procedures or technologies which are not evidence based. For example, in the 1990s, the threat of litigation, often supported by state legislative mandates, forced many health management organisations in the USA to fund high-dose chemotherapy with autologous bone marrow transplants to patients with breast cancer. It was not until 1999 that it became apparent that the procedure provided no benefit compared to standard dose treatment and that in many cases, had caused unnecessary suffering (Deyo 2009). It is therefore important that HTA researchers make a well-argued analysis of the technology under assessment addressing the range of stakeholder perspectives. Partisan views may of course not only come from patients but also from other stakeholders such as clinicians, health technology developers and/or researchers. These kinds of stakeholders may also skew or bias the process.

3.4 Organising Patient Involvement

It is clear that the goals of patient involvement and the choice of participants (patients, patient advocates, caregivers, etc.) will have an impact on the organisation of patient involvement in HTA and the mechanisms for that involvement (Chap. 5). However, the implementation of patient involvement cannot only be concerned with goal setting and choosing the right term. It is also necessary to examine the role of leadership and organisational aspects, particularly the relationship between the participants and the researchers in the HTA or the members of an appraisal committee. Two Danish researchers have suggested a framework of three models of patient involvement, which we briefly describe here. The models are based on research literature about user involvement in healthcare (Holm-Petersen and Navne 2015). Each of the models reflects a different point of departure in relation to the person who is setting the agenda, that is, the doctor, the patient or both of these. We would

argue that these three models may be highly relevant in relation to HTA and patient involvement to understand the relationship between the 'patient' and the HTA researcher (see also Chap. 5 for other models).

3.4.1 Service Minded Involvement

Service-minded involvement positions the *patient as a consumer* and/or the *patient as the public*. This model is in line with new public management ideas which emphasise a business approach to service provision including a focus on customer satisfaction. This may be a relevant model if HTA researchers, for instance, wish to investigate patients' preferences, needs and expectations with respect to a treatment, a new device or a hospital service. We often see this kind of involvement in relation to patient satisfaction surveys, and it is the easiest way of organising patient involvement in HTA, because it is based on 'professional management' (Holm-Petersen and Navne 2015, p. 120). It is the professional—here the HTA researcher—who defines the relationship. However, as we discussed earlier in the chapter, it is not without problems particularly in positioning the patient as 'consumer' (see Sect. 3.2.3).

3.4.2 Supportive Involvement

The supportive involvement position may be understood in relation to *patients as experts, patients as advocates and/or patients as patient representatives*. This kind of involvement supports the patient as an active agent taking part in addressing the needs of a specific group for treatments, investigations and the use of devices (Holm-Petersen and Navne 2015, p. 121) and employing this knowledge in the assessment process. This way of understanding and organising patient involvement in an HTA prioritises patient autonomy, self-care and empowerment. This involvement approach places most of the responsibility on the patients assuming that they know best in relation to their own care. The role of the HTA researcher is then to support, inform and motivate the patient. This form of involvement is based on *patient management* and requires relational work between the HTA researcher(s), the patients and any relatives (Entwistle et al. 2012). However, as we discussed earlier in the chapter (see Sects. 3.2.1, 3.2.2, and 3.2.5), this way of understanding involvement also poses challenges related to which groups of patients will actually be able to be involved. Furthermore in relation to leadership roles and implementation possibilities, many challenges need to be considered, for instance:

- HTA researchers may be reluctant to relinquish control and responsibility
- Patients may come to the HTA process with limited skills to engage in problem definition and discussions due to training, disease burden, physical, psychological and or social-economic reasons (Entwistle et al. 2012, p. 122)

- There may be ethical considerations in relation to leaving a decision to the patient/patient representative (Entwistle et al. 2012)
- There may be an economic challenge related to face-to-face meetings instead of teleconferences (Holm-Petersen and Navne 2015)

3.4.3 Equal Partnership

The idea of equal partnership is especially drawn from the literature examining *shared decision-making*, where both the researcher and the patient contribute with relevant knowledge from their specific perspectives. The agenda is set by the patient and the HTA researcher together, and the relationship between them is based on equality. In relation to patient involvement in HTA, this can be connected to all the terms and to the three goals with patient involvement in HTA we have presented. However, as we especially discussed in relation to the notions of *patients as partisans*, an equal partnership approach also brings many challenges.

3.5 Conclusion

In this chapter, we have reflected on the intricate interrelationship between the terms used to describe patient involvement in HTAs and the goals and framing devices underpinning the involvement processes. We argue that whichever term HTA researchers use and whatever understanding and kind of patient involvement is chosen in relation to a specific HTA, it is essential to engage in critical reflection about terms, goals and the structural organisation of patient involvement in HTA. Part III of this book demonstrates clearly that patient organisations, HTA agencies and HTA researchers are increasingly sensitive to this issue and the needs to provide clear definitions and processes for patient involvement in HTA. This is important in order not to give rise to confusion and potentially dissatisfaction and mistrust between patients and HTA researchers and assessors. Involvement processes developed without due attention to these aspects risk being seen as 'tick-box' processes instituted to give the appearance but not the actuality of patient involvement. Patient involvement in HTA in the future must be more than well-meaning expressions and unreflective use of terms and goals. In particular, HTA researchers have a great responsibility and ethical challenge in being able to reach out to marginalised patients and patient groups who are hard to reach or involve, including patients with acute diseases, homeless people, people with low levels of education or communication difficulties, people with mental disorders and people without social networks. Qualitative and ethnographic research are especially well suited for such research (Chap. 12). Furthermore, there will often (if not always) be a power imbalance among the stakeholders in an HTA with the patients involved sometimes relatively poorly prepared and poorly funded

for the debate compared with clinicians and health technology developers. However, as part III of this book demonstrates, training, mentorship and patient education have received increasing attention in an attempt to address the power imbalance or 'asymmetry of knowledge'. We argue that there is a need for more systematic and rigorous research about the issues discussed in this chapter in order to effectively support the participation of patients and patient representatives in future HTAs.

References

Abelson J, Giacomini M, Lehoux P, Gauvin FP. Bringing 'the public' into health technology assessment and coverage policy decisions: from principles to practice. Health Policy. 2007;82:37–50.

Bastian H. Speaking up for ourselves: the evolution of consumer advocacy in health care. Int J Technol Assess Health Care. 1998;14:3–23.

Batterbury SCE. Evaluation and exclusion from twhe public arena: the case of the British deaf community: chapter 11. In: Boyle R, Breul JD, Dahler-Larsen, editors. Open to the public: evaluation in the public sector. New Brunswick: Transaction Publishers; 2008.

Bridges JF, Jones C. Patient-based health technology assessment: a vision of the future. Int J Technol Assess Health Care. 2007;23:30–5.

Busse R, Oravin J, Velasco M, Perleth M, Drummond M, Gurtner F, et al. Best practice in undertaking and reporting health technology assessments. Working group 4 report. Int J Technol Assess Health Care. 2002;18:361–422.

Dent M, Pahor M. Patient involvement in Europe – a comparative framework. JHOM. 2015;29:546–55.

Deyo RA. Marketing, media, wishful thinking, and conflicts of interest: inflating the value of new medical technology. Permanente J. 2009;13:71–6.

EMA. Revised framework for interaction between the European medicines agency and patients and consumers and their organisations. EMA/637573/2014 adopted. 2014. http://www.ema.europa.eu/docs/en_GB/document_library/Other/2009/12/WC500018013.pdf. Accessed 19 Nov 2016.

Entwistle VA, Firnig D, Ryan M, et al. Which experiences of health care delivery matter to service users and why? A critical interpretive synthesis and conceptual map. J Health Serv Res Policy. 2012;17:70–8.

EUPATI, Patient education!. 2016. https://www.eupati.eu/. Accessed 11 Oct 2016.

Facey K, Boivin A, Gracia J, et al. Patients' perspectives in health technology assessment: a route to robust evidence and fair liberation. Int J Technol Assess Health Care. 2010;70:1518–26.

Facey K, et al. Organisation of services for diabetic retinopathy screening. Health technology assessment report 1. Glasgow: Health Technology Board for Scotland; 2001.

Gallego G, Casey R, Norman R, Goodall S. Introduction and uptake of new medical technologies in the Australian health care system: a qualitative study. Health Policy. 2011;2–3:152–8.

Gauvin FP, Abelson J, Giacomini M, Eyles J, Lavis JN. "it all depends": conceptualizing public involvement in the context of health technology assessment agencies. Soc Sci Med. 2010;70:1518–26.

Gauvin FP, Abelson J, Lavis JN. Evidence brief: strengthening public and patient engagement in health technology assessment in Ontario. Hamilton, Canada: McMaster Health Forum; 2015.

Hansen HP. Evidence-based nursing: must or mantra? In: Kristiansen IS, Mooney G, editors. Evidence-based medicine. In its place. London: Routledge; 2004. p. 33–50.

Harper, D. Online etymology dictionary, 2001–2016. 2016. http://www.etymonline.com/index.php?term=patient&allowed_in_frame=0. Accessed 11 Oct 2016.

Hartzler A, Pratt W. Managing the personal side of health: how patient expertise differs from the expertise of clinicians. J Med Internet Res. 2011;13(3) doi:10.2196/jmir.1728.
Hodgetts K, Hiller JE, Street JM, Carter D, Braunack-Mayer AJ, Watt AM, et al. Disinvestment policy and public funding for assisted reproductive technologies: outcomes of deliberative engagements with three key stakeholders. BMC Health Serv Res. 2014;14:204.
Hofmann B, Saarni SI. Who can and who should represent the patient? Int J Technol Assess Health Care. 2011;27:403.
Holm-Petersen C, Navne LE. Hvad er ledelse af brugerinddragelse? En gennemgang af mål og tre individuelle brugerinddragelsesmodeller. (how to lead patient involvement? Goals and models that organize relations). Bruggerinddragelse Tidsskrift for Forskning i Sygdom og Samfund (J Res Sick Soc). 2015;22:103–33.
HTAi. Interest groups – patient and citizen involvement. 2016a. http://www.htai.org/interest-groups/patient-and-citizen-involvement.html. Accessed 19 Nov 2016 .
HTAi FAQ for HTA agencies and policy makers. 2016b. http://www.htai.org/interest-groups/patient-and-citizen-involvement/resources/for-hta-agencies-and-policy-makers/faq-for-hta-agencies-and-policy-makers.html. Accessed 29 Nov 2016.
Jensen UF. Evidence, effectiveness and ethics: cochrane's legacy. In: Kristiansen IS, Mooney G, editors. Evidence-based medicine. In its place. London: Routledge; 2004. p. 20–33.
Keating P, Cambrosio A. Cancer on trial. Chicago: University of Chicago Press; 2012.
Kristensen FB, Sigmund H, editors. Health technology assessment handbook. Copenhagen: Danish Centre for Health Technology Assessment, National Board of Health; 2007.
Liberati A, Sheldon TA, Banta HD. Eur-ASSESS project subgroup report on methodology: methodological guidance for the conduct of health technology assessment. Int J Technol Assess Health Care. 1997;13:186–219.
Lopes E, Carter D, Street JM. Power relations and contrasting conceptions of evidence in patient involvement processes used to inform health funding decisions in Australia. Soc Sci Med. 2015;135:84–91.
Menon D, Stafinski T. Role of patient and public participation in health technology assessment and coverage decisions. Expert Rev Pharmacoecon Outcomes Res. 2011;11:75–89.
Royle J, Oliver S. Consumer involvement in the health technology assessment program. Int J Technol Assess Health Care. 2004;20:493–7.
Russell J, Greenhalgh T. Being rational and being 'human': how national health service rationing decisions are constructed as rational by resource allocation panels. Health. 2014;185:441–57.
Street J, Braunack-Mayer AJ, Ashcroft R, Facey K, Hiller J. Virtual community consultation? Using the literature and weblogs to link community perspectives and health technology assessment. Health Expect. 2008;11:189–200.
Tritter JQ. Revolution or evolution: the challenges of conceptualizing patient and public involvement in a consumerist world. Health Expect. 2009;12:275–87.
Van der Weyden MB, Armstrong RM. Evidence and Australian health policy. MJAFI. 2004;180:607–8.

Chapter 4
Patient-Based Evidence in HTA

Sophie Staniszewska and Sophie Werkö

4.1 Introduction

This chapter provides an introduction to patient-based evidence, which can be based on patients' experiences, perspectives, perceptions, needs, preferences or attitudes about their care and health (Staniszewska et al. 2010). It explores the concept of patient-based evidence and its role in HTA and compares it to patient input. Some of the challenges that arise about patient-based evidence due to the sometimes rigid application of evidence hierarchies designed for clinical research are considered. We review case studies where patient-based evidence has been used in HTA and consider how misconceptions about patient-based evidence might be overcome in order to encourage a greater use of the concept in HTA. Barriers and enablers to the development and use of patient-based evidence are suggested that require action by all stakeholders. We note the importance of co-production in the development of patient-based evidence and the need for further research to develop patient-based evidence as a concept.

This chapter presents arguments that patient-based evidence, which can be produced from a range of research genres, is an important type of knowledge, which should generally be included in HTA. We understand patient-based evidence as a complex concept, which we will expand conceptually and theoretically in a companion paper (Staniszewska and Werko 2017).

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In terms of research genres, methodologies and methods, patient-based evidence can be produced using qualitative and quantitative approaches (Staniszewska et al. 2014; Staniszewska et al. 2010). It is important to acknowledge that patient-based evidence is not the same as information that comes to HTA via the participation of patients (or patient groups or informal caregivers) in the HTA process (Chap. 5). Patient-based evidence and the information that patients contribute directly to HTA may be seen as complementary, while in other HTAs they may be opposed to each other. The key distinction is that the information which patients contribute as they participate in an HTA process does not usually originate in research, although it can, but in the range of perspectives that individuals or organisations bring. However, patient input can help with the assessment of patient-based evidence, for instance, in the Swedish examples in Chapter 28, when patient input confirmed the patient-based evidence. Table 4.1 summarises some of the differences between the two concepts.

Patient-based evidence can inform colloquial evidence. Colloquial evidence can be defined as an umbrella term consisting of different types of informal expert opinion from clinicians and patients, their views and stories (Sharma et al. 2015; Lomas et al. 2005).

Table 4.1 A summary of patient-based evidence compared to information from patient participation in the HTA process

Patient-based evidence	Patient participation in the HTA process
Produced through research, generally published in peer-reviewed journals	Originates in perspectives of individuals, groups of patients or organisations
Draws on a range of research genres and methodologies	Does not necessarily use or need a specific methodology
Draws on robust scientific methods whose strengths and limitations are known and provides a robust conclusion that can be clearly interpreted	The quality of the methods used to gather inputs may be unclear or not considered as important
Depends on appraisal of quality including formal critical assessment and peer review	The concept of quality may depend on factors such as authenticity or diversity of perspectives
Research is based on specific research questions and takes time to generate from either primary or secondary research	Patient participation can be used at any point in the HTA process and may be in the form of a dialogue to enable immediate reaction to an emerging issue
May be more limited in accounting for context of the HTA, depending on whether studies have considered context	Can consider the context of the HTA question
Can be based on a synthesis of studies which means a comprehensive, unbiased view of a patient issue can be summarised very effectively	Provides a selection of perspectives which may not be comprehensive but informative
Research directly addresses questions of bias and balance, which provides some assurance of quality	Bias in relation to patient input is a complex concept that requires exploration in the future

Patient-based evidence is based on research sometimes generated collaboratively with patients and sometimes by researchers. It is most often published in peer-reviewed journals. However, it is unclear whether all patient-based research conducted by commercial companies is eventually published in peer-reviewed journals. In the future, it would be helpful if all patient-based evidence was published in a transparent and accessible way, which would avoid waste in research. This means that patient-based evidence stems from approaches including patient-reported outcomes (Chap. 9), discrete choice experiments (Chap. 10), analytical hierarchy processes (Chap. 11), ethnographic fieldwork (Chap. 12), deliberative inclusive methods (Chap. 13) and synthesis of qualitative research (Chap. 15).

Patient-based evidence may be very powerful when based on a synthesis of either qualitative, quantitative or mixed method studies. In any type of synthesis, it is important that the primary studies have been conducted with high methodological quality and that all studies are assessed systematically and synthesised appropriately. Patient-based evidence can play a crucial role in achieving a comprehensive or full HTA (Chap. 1). However, we argue that despite early recognition of the importance of patient-based evidence in HTA, the focus on economic methods and rapid HTA tends to diminish the focus on patient-based evidence at a time when it is becoming increasingly important. This jeopardises the idea of a comprehensive HTA where all relevant aspects of the appraisal of a technology are considered, particularly that of the patient.

We note examples of the use of patient-based evidence in HTA, including, by Sweden (Chap. 28), Denmark (Chap. 22), Scotland (Chap. 27) and Germany (Chap. 25), organisations that have taken important steps in broadening their vision of what evidence is required for HTA (Staniszewska et al. 2014). In the future, it would be a good practice for HTA organisations to always report the patient-based evidence. Importantly, we argue patient-based evidence should have the same status as clinical and economic forms of evidence.

4.1.1 Exploring Patient-Based Evidence

In order to explore patient-based evidence for HTA, we need to consider the concept of evidence, the appraisal of which forms the cornerstone of HTA (Merlin et al. 2009). A key factor underpinning high quality evidence is its validation and verification through scrutiny (Davies et al. 2000). Evidence is often assumed to be research based and quantitative (Sackett et al. 1997). Hierarchies of evidence relating to clinical research have been established that place systematic review of all randomised controlled trials at the highest level, followed by evidence obtained from at least one properly designed randomised controlled trial (Guyatt et al. 2000). Over time this hierarchy has been applied to HTA (Merlin et al. 2009), not just to clinical effectiveness questions but also to other research questions, such as those relating to patient aspects.

The focus on quantifiable research-based evidence informed the development of evidence-based medicine and thus evidence-based practice, defined by Sackett et al. (1996) as 'the conscientious, explicit and judicious use of current best evidence about the care of individual patients'. Sackett et al. (1996) stated that the practice of evidence-based medicine means integrating individual clinical expertise (which includes a consideration of patients' preferences and perspectives) with the best available external clinical evidence from systematic research. By best available clinical evidence, they referred to clinically relevant research, often from the basic sciences of medicine, but 'especially from patient-centred clinical research' although the exact nature of this form of evidence was not specifically defined. While we support Sackett et al.'s (1996) initial emphasis, we are aware that they did not develop patient-based evidence as a substantive concept in itself. Rather they suggested that patients' perspectives should be considered in a clinical encounter, changing this to a form of individual input not necessarily viewed as evidence. While not directly applicable to HTA, we draw on this as an example of where key concepts used in HTA have emerged from. We argue that there is an important need to address this gap in our conceptualisation of evidence and to formally consider patient-based evidence alongside clinical and economic evidence.

While we refer to the concept of patient-based evidence in this chapter and attempt an initial definition earlier in the chapter, it is important to acknowledge that the term has not been commonly used or defined adequately in the literature or in HTA. Patient-based evidence is a term that was used by a module of the Oxford University Masters in evidence-based healthcare,[1] which ran for nearly a decade and was linked to the evidence-based medicine movement promoted by Sackett during his time in Oxford. A paper used the term to describe the development of a patient-based evidence base in chronic fatigue syndrome, focusing on experiences of the condition and health service experiences as key sources of evidence for practice (Staniszewska et al. 2010). More recently, patient-based evidence was conceptualised as the key form of evidence underpinning the NICE patient experience guidance and quality standard (Staniszewska et al. 2014). These papers described patient-based evidence as including information about patient experiences, perceptions, needs or attitudes about their care and health. It could also include patient narratives, data on health-related quality of life, data on quality-adjusted life years or published patient experiences' survey data (Staniszewska et al. 2010).

In addition, patients' preference is an area of research which is currently being developed in several places. One such European initiative is the IMI project PREFER that started in October 2016 and will run for 5 years. PREFER aims to develop recommendations about the elicitation and use of patient preference data in the assessment of the benefits and risks of medicinal products, to inform decision-making processes by regulators and HTA bodies (PREFER 2016).

[1] Led by Sophie Staniszewska

While patient-based evidence is not an accepted or a necessarily recognised term in HTA, aspects of it have, to some extent, been included in HTA in different ways. However, more needs to be done to consider how it can be best generated and used to inform HTA.

There are important examples of its application and use in HTA and related areas. Several HTAs produced by the Swedish Agency for HTA and Assessment of Social Services (SBU) provide examples of patient-based evidence using qualitative research methods to synthesise patient experiences (Chap. 28). These examples all focus on how people perceive and experience their condition, their health and their quality of life and what this means to them. Before synthesis, published scientific studies are critically assessed to determine their relevance and quality using assessment tools that are specific to qualitative research. Only studies of moderate and high quality are then included in the qualitative evidence synthesis. This creates robust patient-based evidence that has undergone a scientific process, similar to the other evidence in HTA, but which uses a very different understanding of the hierarchy of evidence.

An example of an international development in patient-based evidence is INTEGRATE-HTA (INTEGRATE-HTA 2013), a three-year project that focused on the development of concepts and methods that enable a patient-centred, comprehensive assessment of complex health technologies, using palliative care as a case study (Wahlster et al. 2016). It has created a structure for assessments of complex technologies, which takes context, implementation issues and patient characteristics into account. It aims to make the HTA or systematic review more meaningful for real-life decision-making, and it addresses how to generate patient-based evidence. Further examples of the use of patient-based evidence are given in Parts II and III of this book. In developing patient-based evidence within HTA, we need to involve those with social science expertise in the development and use of methods and methodologies, both qualitative and quantitative, in HTA.

4.2 Barriers to Integrating Patient-Based Evidence

There are a range of barriers to identifying and integrating patient-based evidence in HTA, including its status, conceptual and methodological challenges and the paucity of approaches to the integration of patient-based evidence alongside clinical and economic evidence. We summarise each of these areas identified based on the authors' joint experience and expertise in Table 4.2.

4.3 Enablers to Integrating Patient-Based Evidence

A range of potential enablers for identifying and integrating patient-based evidence into HTA can be identified on the basis of the authors' joint expertise, as outlined in Table 4.3.

Table 4.2 Barriers to patient-based evidence

Barrier	Issue
Knowledge about patient-based evidence	Researchers and clinicians sometimes have a modest understanding of patient-based evidence
Status of knowledge	Patient experiences and views have been viewed as anecdotal and of low quality
Leadership	The people leading development of patient-based evidence often have a social science background and when compared to clinical leaders may be perceived to be of lower status in terms of capacity to influence and effect change
Types of HTA question	The hierarchy of evidence that HTA uses often privileges clinical and economic questions and may not recognise that patient-based evidence needs to be in a different form
Concept development	Weak conceptualisation of patient-based evidence creates difficulty in its identification and application
Methods development	Patient-based evidence as a scientific area lacks the strategic and focused effort and support provided to other areas of methods development
Integration of patient-based evidence with other forms of evidence	Methodologically, although there has been progress and a range of qualitative and quantitative approaches exist, there has been less progress on methods that enable integration of patient-based evidence alongside clinical and economic evidence
Capacity to undertake high quality research to generate robust patient-based evidence	HTA bodies appear keen to employ economists, but those from social science backgrounds that could promote the value of and undertake research into patient aspects is needed
Robust patient-based evidence	There is a need to discuss the types of research that would generate robust evidence for HTA decision-making, including the role of context, as discussed in Part II

Table 4.3 Enablers to patient-based evidence

Enabler	Potential solution
Paradigm shift	There is a need for the HTA community to recognise the importance of paradigm change in how we conceptualise the nature of research evidence for HTA
Raising the status of patient-based evidence	The status of patient-based evidence as an equally important form of knowledge alongside clinical and economic evidence needs to be recognised
Informing governments, policy makers and decision-makers	Governments, policy makers and decision-makers need to be informed about patient-based evidence so they can demand the changes needed to develop the patient-based evidence for HTA
Ensuring funding is available	Research, HTA funders and industry have an important role in funding work that generates patient-based evidence
Conceptual and methodological development	Patient-based evidence is a neglected asset and requires development
Supporting the HTA community, patients and patient organisations	Researchers, clinicians and patients need to work together to demonstrate the benefits of patient-based evidence

4.4 Patient Involvement in the Creation of Evidence

In calling for greater effort to develop the concept of patient-based evidence in HTA, we recognise that we need to develop new approaches and methods, co-produced with patients and the public. The term co-production highlights the potential relationships that could exist between the producers and 'clients' when it was realised that the production of a service was difficult without the active participation of those intended to receive it (Ostrom 2002). While patients are vital as 'suppliers' of patient-based evidence, through their inclusion as subjects in studies, they also have a fundamental role to play as active collaborators in creating patient-based evidence conceptually and methodologically (Stephens and Staniszewska 2015). Such co-production of patient-based evidence will require new ways of working, cultures and systems that support this, reflecting the ethos of public involvement practice (Wilson et al. 2015).

4.5 Conclusion

This chapter has introduced the concept of patient-based evidence in HTA and suggested an initial definition. There is a need to develop this concept theoretically and methodologically and to include patient-based evidence in the HTAs, to ensure that robust research on patient perspectives, experiences, etc. become addressed. We have now reached a tipping point. While we recognise the important progress made to date in HTA, we urge the HTA community to join in a unified effort to ensure HTA is even more fit for purpose for the twenty-first century by embedding patient-based evidence in its work. The work of the HTAi Patient and Citizen Involvement Interest Group will also be taking this endeavour forward.

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References

Davies H, Nutley S, Smith P. Introducing evidence based policy and practice in public services. In: Davies H, Nutley S, Smith P, editors. What works? Evidence based policy and practice in public services. Bristol: The Policy Press; 2000. p. 1–11.

Guyatt GH, Haynes RB, Jaeschke RZ, Cook DJ, Green L, Naylor CD, et al. Users' guides to the medical literature XXV. Evidence-based medicine: principles for applying the users' guides to patient care. JAMA. 2000;284:1290–6.

INTEGRATE-HTA. 2013. http://www.integrate-hta.eu. Accessed 1 Nov 2016.

Lomas J, Culyer AJ, McCutcheon C, McAuley L, Law S. Conceptualizing and combining evidence for health system guidance. Ottawa: Canadian Health Services Research Foundation; 2005.

Merlin T, Weston A, Tooher R. Extending an evidence hierarchy to include topics other than treatment: revisiting the Australian 'levels of evidence'. BMC Med Res Methodol. 2009;9:34.

Ostrom E. Crossing the great divide: coproduction, synergy, and development. In: McGinnis MD, Ostrom E, editors. Polycentric governance and development: readings from the workshop in political theory and policy analysis. Michigan: The University of Michigan Press; 2002. p. 346–74.

PREFER: Patient preferences. 2016. http://imi-prefer.eu. Accessed 30 Nov 2016.

Sackett DL, Rosenberg WMC, Gray JA. Evidence based medicine; what it is and what it isn't. Br Med J. 1996;312:71–2.

Sackett DL, Richardson WS, Rosenberg W, Haynes RB. Evidence based medicine. How to practice and teach EBM. Edinburgh: Churchill Livingstone; 1997.

Sharma T, Garner S, Kaur B, Choudhuri M, Naidoo B, Littlejohns P, Staniszewska S. Evidence informed decision-making: the use of 'colloquial evidence' at NICE. Int J Qual Health Care. 2015;31:1–2.

Staniszewska S, Werko S. Some of our evidence is missing: conceptualizing and using patient-based evidence in HTA. (forthcoming) 2017.

Staniszewska S, Boardman F, Gunn L, Roberts J, Clay D, Seers K, et al. The Warwick patient experiences framework: patient-based evidence in clinical guidelines. International Journal of Quality in Health Care. 2014;26:151–7.

Staniszewska S, Crow S, Badenoch D, Edwards C, Savage J, Norman W. The PRIME project: developing a patient-based evidence-base. Health Expect. 2010;13:312–22.

Stephens R, Staniszewska S. One small step…. Research Involvement and Engagement. 2015;1:1.

Wahlster P, Brereton L, Burns J, Hofmann B, Mozygemba K, Oortwijn W et al. Guidance on the integrated assessment of complex health technologies – the INTEGRATE-HTA model. 2016. http://www.integrate-hta.eu/downloads/. Accessed 1 Sept 2016.

Wilson P, Mathie E, Keenan J, McNeilly E, Goodman C, Howe A, et al. ReseArch with patient and public invOlvement: a realisT evaluation – the RAPPORT study. Journal Health Services and Delivery Research. 2015;3 doi:10.3310/hsdr03380.

Chapter 5
Developing the Mosaic of Patient Participation in HTA

Karen M. Facey

5.1 Introduction

In this book, patient involvement has been defined as encompassing research to produce patient-based evidence and participation of patients in HTA (Chap. 1). This chapter explores potential processes for patients to participate in HTA. It reviews hierarchies that characterize public participation in policymaking from minimal to empowering. Then participation approaches that relate specifically to health policy, HTA and patients are considered. The challenges and enablers to support patients (and patient groups and informal caregivers) to participate in HTA are explored. Finally, mechanisms of patient participation that an HTA body could use are presented for each stage of HTA, covering not just individual HTAs but also HTA process and high-level HTA policy. This creates a mosaic of patient participation in HTA that is presented for discussion and most importantly as an aid to guide HTA bodies in establishing optimal processes for patient participation in HTA in their own setting.

5.1.1 Defining Participation

Depending on the field of study and country of origin, terms for participation vary, and in some cases the terms may be interchangeable, and in other cases one term may be a subset of another. This is shown in the work of the International Association of Public Participation, where involvement is in the middle of their spectrum of participation (IAP2 2016). As outlined in Chap. 1, *involvement* is used as an overarching

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term in this book, a subset of which is *participation*. Hence, when describing notions from published references in this chapter, terms may have been altered to ensure clarity in this book.

5.1.2 The Case for Patient Participation in HTA

For decades, patients have worked together in communities to raise public awareness of their disease, promote education and enhance political activism (Maxwell 2015). Some of the early examples were in the field of cancer, where the Leukemia and Lymphoma Society was founded in 1944 by a family in memory of their son (Maxwell 2015) and in AIDS, where a variety of patient groups were established in the early 1980s as the disease emerged (Royles 2012).

Over the past 15 years, as HTA has been used to inform decisions about treatment reimbursement and access, patient groups have advocated that patients' perspectives should be fully understood in the deliberative appraisal process in HTA. At the same time, many HTA bodies have had to streamline their work to produce reports faster, leading to reduced resources for research to develop patient-based evidence (Chap. 1). Thus, new ways have needed to be developed to understand patients' perspectives, mainly via participation activities that fit within short HTA timelines.

Academic arguments from ethics, philosophy of science, political science and social science have legitimized patient participation in HTA (Gauvin et al. 2011). However, many HTA bodies still have concerns about patient participation in HTA, including that patient input is anecdotal and biased due to conflicts of interest (Facey and Hansen 2011).

This chapter explores how patients can be supported to participate in HTA to contribute their perspectives in a manner that will add value to the largely scientific process of HTA. While recognizing the important differences between patient and public participation, I will review some relevant frameworks for both, before considering specific approaches to patient and public participation in HTA and then focus on patient participation in HTA.

5.2 Conceptualizing Participation

Over a decade ago, Rowe and Frewer (2005) stated that public participation was imprecisely defined, used in many contexts, had different aims and could take many forms. A more recent systematic review confirmed that the conceptualization of patient participation in healthcare and health research is still vague, has changed over time and offers a fragmented and partial vision (Barello et al. 2014).

One of the earliest frameworks for participation was the ladder of citizen participation in planning policy (Arnstein 1969). The ladder had eight rungs, with manipulation and therapy at the bottom indicating non-participation; then informing, consultation and placation indicating tokenism; and partnership, delegated

power and citizen control indicating citizen power. This ladder of participation is often reproduced as the most relevant treatise on the subject of patient participation in health service research and HTA. However, Arnstein recognizes that her model is an oversimplification, with more rungs that are less distinct than the ladder analogy suggests (Arnstein 1969).

Over 30 years later, Tritter and McCallum (2006) noted that the participation ladder is a hierarchical entity based on a conceptualization of activism, with control/power at the top. Tritter (2009) reflected that the ladder model assumes power is finite and that ceding power to one group diminishes power in another. His alternative view was that there are different kinds of knowledge and power in health policy and that partnership and collaboration can result in a better outcome (see also Chap. 3). The goal is not to hand over power but to support sharing of knowledge and expertise from a range of perspectives to inform the design of services. Such a participatory process is complex and evolutionary in nature and should be negotiated with all stakeholders. This knowledge-sharing approach would seem a better fit for HTA as the 'power' is actually in the hands of the health system who make the final decisions about the availability of health technologies based on the HTA recommendation.

Boivin et al. (2014) noted that in health system planning decisions, public participation that merely provides a 'seat at the table' for a representative, without appropriate support, is unlikely to change healthcare policy decisions (tokenistic participation). They referenced three main constructs to explain the public's influence on collective decisions:

- Credibility (technocratic processes): the ability to contribute knowledge that is considered valid and relevant and will result in mutual learning and generation of new solutions.
- Legitimacy (democratic processes): to speak on behalf of people affected by health services.
- Power and ability to influence.

They concluded that participants need to be supported to become a credible, legitimate, powerful source of knowledge for professionals, to be seen as 'experience-based experts'.

In HTA, the work to conceptualize patient/public involvement has been undertaken by a range of researchers in Canada, where authors tend to favour the typology developed by Rowe and Frewer (2005). Adapting their terminology to that used in this book, they categorize participation based on the direction of information flow as:

- Communication (decision maker ⇒ patients).
- Consultation (decision maker initiates process: patients ⇒decision maker).
- Dialogue (decision maker ⇔ patient).

Two-way dialogue transforms the opinions of both the decision makers and patients, but its effectiveness depends on the fairness of the mechanism of dialogue and its competence and efficiency in achieving its intended purpose. This requires a serious intent to maximize the relevant contribution (knowledge/views) from the maximum number of sources and depends on the selection of those involved, form of information elicitation and information sources. Then that input must be

processed to ensure minimal information loss when combined with other information. All these issues must be considered to ensure patient participation in HTA is optimized.

5.3 Reviewing the Development of Patient and Public Participation in HTA

In 1998, the International Journal of Technology Assessment in Health Care (IJTAHC) published a themed issue entitled The Consumer and Technology (IJTAHC 1998). The articles covered issues relating to patient advocacy/empowerment in healthcare, comparisons of quantitative methods to elicit patients' perspectives and communication of evidence-based information. They were meant to explore the developments in these fields and their implications for HTA, but focus appeared to be more on communicating HTA to patients and issues of HTA process (disclosure) rather than encouraging patient participation.

In 2004, Coulter expressed disappointment about a series of articles in IJTAHC presenting national approaches to HTA, which seemed to imply that patients were peripheral to the HTA process. She encouraged patient participation in all stages of HTA and proposed that HTAs should start with the types of questions that patients ask their clinicians (including characteristics of disease, trade-offs between length of life, quality of life and different forms of treatment) and the outcomes that are important to them.

In 2005, some progress was made, with two important international initiatives: HTAi established its Interest Group for Patient/Citizen Involvement in HTA (the HTAi Interest Group), and the International Network of Agencies for HTA (INAHTA) undertook a survey and discussion paper of patient/citizen involvement in HTA bodies. It noted that decisions about healthcare priorities depend on value judgements, and so all decision-making processes should be open, transparent and inclusive (Hailey 2005). Hailey concluded that HTA needed to give more attention to the views of patients as the ones directly affected by the technology being assessed, but that approaches to patient involvement would depend on the mandate, governance and resources of each HTA body and the particular technology being assessed. Bridges and Jones (2007) noted that HTA had evolved from the traditional evidence hierarchies of evidence-based medicine to include more diverse components such as economics and quality of life. Bridges and Jones (2007) suggested that patients needed to be partners in HTA, to enable HTA to be more patient-focussed, taking account of patients' perspectives and preferences alongside the other aspects considered in an HTA.

Despite this progress, Gauvin et al. (2010) identified the struggle that many HTA bodies had had in implementing patient/public participation in HTA due to concerns about scientific integrity. They reported mixed methods research, which identified that patient/public participation in HTA depended on:

- The technology being assessed.
- The institutional context of the HTA body.

- The interests of those who want to be involved vs those of the HTA body.
- Whether patient/public input is deemed to provide legitimate evidence.

Surveys (Hailey et al. 2012, Whitty 2013, EPF 2013)[1] and a systematic review and interviews (Menon and Stafinski 2011) have been undertaken with HTA bodies about patient (and public) involvement in recent years. The surveys had responses from 33 to 40 HTA bodies internationally. In the INAHTA survey (Hailey et al. 2012), 22 HTA bodies (67% of responders) involved patients and the public, whereas in the EPF survey (2013), 18 (45%) stated that they involved patients. The type and level of participation varied widely across the stages of HTA, but the majority stated that patient groups could participate in topic selection, provide input to assessments and review draft recommendations. However, fewer HTA bodies presented or integrated patients' perspectives in their reports, produced patient-friendly HTA report summaries or conducted evaluations of the impact of patient participation. The picture is of course worse than presented, as presumably those not involving patients did not respond to the surveys.

In 2011, the HTAi Interest Group led a special themed edition of IJTAHC that included papers about research into patient aspects, patient participation in HTA programmes and the need to demonstrate the impact of patient involvement in HTA (IJTAHC 2011).

Gagnon et al. (2011) suggested that including experiential evidence from patients in HTA could allow a more accurate assessment of the value of health technologies. Menon and Stafinski (2011) also suggested that in an era of incremental health gains, patient insights about the relative value of a health technology could be increasingly important, but this could challenge decision makers who rely on traditional forms of evidence to assess value. They concluded that it is essential to use robust methods to collect patients' perspectives in HTA (Menon and Stafinski 2011).

A more recent literature review (Gauvin et al. 2015) still found that it was difficult to identify what meaningful patient/public participation in HTA was from the literature, given:

- Divergent views about what patient participation means.
- Lack of research about effectiveness and impact.
- Uncertainty about most appropriate way to integrate patient participation with other types of evidence.

However, the evidence confirmed that the case for patient/public participation in HTA was strong and could achieve the following goals:

- Democratic.
- Scientific (including patient-based evidence).
- Instrumental (better quality decisions at each stage of HTA).
- Developmental (increasing awareness of HTA and building capacity to contribute).

[1] European Patients' Forum

These and other ethical rationales for patient participation in HTA are critically explored in Chap. 2.

5.4 Barriers and Enablers to Patient Participation in HTA

Patient participation in HTA is still contentious (Gauvin et al. 2010), with differing views about who should be involved, when and in what way. The scope and type of patient/public involvement varies according to the power and authority (within or outside government, advisory recommendations, binding decisions on reimbursement, etc.) and the policies of the organization that hosts the HTA body (Gauvin et al. 2011, Kreis and Schmidt 2013). Gauvin et al. (2015) noted that in light of resource and institutional constraints, determining which methods to use at which stage of HTA is an elusive goal. Therefore, the purpose of this section is to explore why patient participation in HTA is still contentious and identify the barriers and enablers to participation in HTA. The next section will then seek to address the when, why, who and how of patient participation with the overarching purpose of adding value to HTA.

Hailey (2005) presented barriers to patient/public involvement in HTA that appeared still extant a decade later (Facey and Stafinski 2015). These are presented in modified form in Table 5.1, updated with additional review references.

Table 5.1 Barriers to patient participation in HTA (Adapted from Hailey 2005)

Challenge	Issues
Interaction of consumers and researchers	Time needed to develop a trusting, productive relationship
	Concerns about attitudes of HTA body (tokenism) or patient group (those with strongly held beliefs less willing to be constrained by research evidence)
	Concern about objectivity of patient group if they have received funding from health technology developers
Resources	Administrative
	Financial
	Support staff
Mechanism of participation	Lack of comprehensive approach that sets goals of participation for each stage of HTA (Gauvin et al. 2015)
	Often chosen by the decision maker, who shapes it in a specific manner and so has control over the participation (Boivin et al. 2014)
Identifying a 'patient position'	Recognizing that there are differing values, expectations, environment, culture, genetics and experiences of patients in the health system, but that it is not possible to canvass all
Nature and extent of patient representation	Difficulty defining which patients should be involved
	Questions about representativeness
	Concerns about conflicts of interest and influence of health technology developers
	Difficulty reaching marginalized populations

Table 5.1 (continued)

Challenge	Issues
Technical demands	Lack of knowledge/power/credentials/skills in scientific process and healthcare policy options
	Impact of declining health condition on ability of patient to contribute
Training and education	Lack of education and training developed specifically for patients
Time demands and remuneration	Time commitments
	Working to tight timetables
	Payments that should be made to patients
Balancing information from researchers, the literature and patients	Lack of concordance between issues that patients regard as important and those in which research has been conducted
	Concern about methodology to balance qualitative and quantitative evidence and the role of costs, including questions about credibility of patient-based evidence
	Devaluing of patient-based evidence in evidence hierarchies (Gauvin et al. 2011)
Use of patient input	Unsure what to do with patient representatives or how to involve them
	Concern of tokenism
	May increase time needed for appraisal
	Poorly moderated discussions may not enable patients to contribute (Boivin et al. 2014)
	Concerns by researchers/clinicians that scientific debate gets softened by inclusion of patients' perspectives
	Possible distortion of funding decisions due to patients' biases
	Patient groups concerned about how evidence from different sources is handled, weighed and valued and that others have more influence
	Power differences between patients and professionals (Boivin et al. 2014)
	Conflicts of interest (Facey and Hansen 2011)
Lack of awareness of HTA processes	The implications of HTA processes are not understood
	Patients do not know how HTA is used or how to participate
Few evaluations of patient involvement	Absence of good quality research to show that patient involvement makes a difference
	No demonstration that patient involvement improves quality of assessments (Gauvin et al. 2015)

Where politics and science meet there will always be difficulties. However, despite the list of barriers being long, HTA bodies are developing processes to support patients to participate in HTA. These are captured in Table 5.2 according to the HTA contexts outlined in the *HTAi Values and Quality Standards for Patient Involvement in HTA* (Sect. 1.4 in Chap. 1), with the addition of the policy context identified by Gauvin et al. (2010).

Although these barriers and enablers have been written from the perspective of HTA bodies, many are also relevant for patient groups.

Table 5.2 Enablers of Patient Participation in HTA

Context	Enabler	Description
Policy	Cultural and political context	National/regional policies that encourage patient participation in health service design and delivery
	Critical mass of HTA bodies	Patient participation in HTA is considered the norm, with processes that can be shared and adapted
	Leadership	HTA and political leaders encourage/mandate HTA bodies to involve patients
		HTA bodies are reviewed by their funders about the effectiveness of their mechanisms for patient participation
HTA process	Rationale articulated	Identify principles, objectives and goals for patient participation at each stage of HTA process
	A range of mechanisms	Offer different approaches depending on stage of assessment and goal
		Decide whether to involve individual patients, caregivers, patient representatives or patient groups
	Evaluation	Review participation approaches against intended goals
		Monitor satisfaction of patients who are involved
		Modify processes with feedback from all stakeholders
		Innovative culture to develop processes for participation
Individual HTAs	Careful recruitment and support	Clear recruitment strategies
		Presence of patient group that can represent users of the health technology
		Structured training and support
		Opportunities for patients to interact with one another
		Buddying with another patient representative
		Dedicated staff contact
		Mentoring
		Payment to prepare materials and attend meetings
	Sufficient time	Advance notice of upcoming HTAs that might be of relevance
		Sufficient notification in the HTA process to allow patients and patient groups to prepare their input in collaboration with others
	Appropriate materials	Technical information conveyed understandably
		Administrative support (printing papers)
	Inclusive meetings	Preparatory meetings to help discuss areas where patient contributions would be most valuable
		Appropriate timing and setting of meeting to suit patients' condition
		Chairing by an expert moderator to enable everyone to be heard in interdisciplinary discussions
	Clear reporting	HTA report presents how patients were involved and the difference they made

Gauvin et al. 2011, OHTAC 2015 (Ontario Health Technology Advisory Committee), Boivin et al. 2014, Kreis and Schmidt 2013, Facey and Hansen 2011

5.5 Developing the Mosaic for Patient Participation in HTA

In addition to the enablers presented in Table 5.2, there are overarching issues to consider such as who to engage, when and how. As discussed previously, this is complex and cannot be addressed by a simple ladder or one size fits all approach. A multidimensional framework is needed that can be drawn upon by any HTA body, whatever their context or current processes.

As an alternative to Arnstein's participation ladder, Tritter and McCallum (2006) suggested a two-dimensional 'mosaic' of participation to allow mapping and monitoring of patient participation, based on methods of participation, category of health service user and outcomes. Gauvin et al. (2010) used this concept to develop a framework for patient/public participation in HTA outlining eight levels of involvement for six 'publics' at each stage of HTA.

Later, Gauvin et al. (2015), OHTAC (2015) and Gagnon et al. (2015) stressed the need to also be explicit about the goals of patient and public involvement. Interpreting this work specifically for patients seems to imply that the goals for patient involvement in HTA are:

- Quality of scoping and recommendations improved by incorporating patients' values and experiences.
- Clinical evidence review is enriched through focussed attention on values and perspectives of priority populations.

Menon et al. (2015) went further and stated that patient involvement is likely to have most impact if it can reduce uncertainties in assessment. They define these 'decision uncertainties' as determining clinical benefit, value for money, affordability and adoption (e.g. treatment stopping rules).

So to develop a mosaic for patient participation in HTA, Gauvin's framework could be modified to use the HTA contexts presented in Table 5.2, identify the specific HTA stages within these levels and add goals. Then, rather than depicting general levels of involvement as outlined by Gauvin et al. (2010), specific mechanisms for patient participation can be suggested, as shown in Table 5.3. This table has been developed in consultation with the authors in Part III of this book, who have suggested the wide range of mechanisms presented.

The mosaic in Table 5.3 is complex but presents a wide range of mechanisms that HTA bodies can use to support patients to participate in their processes and as such is generic. It is not expected that any HTA body undertakes all aspects. This is not a statement of best practice or call for universalization of particular mechanisms of participation. An HTA body would be expected to use it for reference and create its own mosaic—identifying the steps in its process, why it wants patient participation at each step, who it will involve and how. Chapter 25 shows how the mosaic can be used by an HTA body.

Not all of the potential mechanisms of participation are widely used in HTA. The ones that seem to be growing in popularity are those we may call 'patient input', where the burden of participation lies on the patient or patient group. They actively

Table 5.3 Mosaic of patient participation in HTA

Phase (stage)	Why (goals/values)	Who to involve	How—mechanisms that HTA body could organise
Policy			
Public sector funding decision for research to address uncertainties (before or after HTA)	Relevance	Patient groups	Participation in decision-making committee or other forms of priority setting process for defining research priorities for public funding
		Individual patients	
Development of HTA processes	Relevance	Individual patients	Consultation/research on assessment methods
		Patient groups	
	Fairness Legitimacy	Patient groups	Workshops and use of feedback from patient groups to develop patient participation methods
	Relevance Fairness Legitimacy	Patient groups	Formally evaluate and research patient participation methods
	Fairness	Patient groups	Publish a policy for patient participation that indicates patients' rights and process for participation
HTA process			

Proposal of HTA topics	Relevance	Patient groups	Submission of potential topics online or via paper form (with support from HTA researchers to complete the form)
	Relevance	Individual patients[a]	Group discussion (focus group, Delphi, etc.) with HTA researchers to identify potential topics
	Fairness	Patient groups	Public consultation on proposed topics and policy questions
	Relevance	Patient group member	Patient representative on selection committee for a themed call of topics
Communication	Fairness	Patient groups	Notification of timelines for an HTA including points at which patient groups can participate
	Fairness	Patient groups	Accessible reports and communication methods that take account of the limitations of the condition and possible comorbidities
	Legitimacy	Individual patients	Media campaigns to communicate the HTA recommendations
Building capacity for patients to contribute	Building capacity Fairness	Patient groups	Dedicate HTA staff to work on patient involvement and provide patient groups with a named individual to contact
	Building capacity	Patient groups	Feedback in person or writing on submission to HTA committee
	Building capacity	Individual patients	Deliver training courses led by HTA staff
		Patient groups	
	Building capacity	Patient groups	Contribute to training courses developed by patient groups
	Building capacity	Patient groups	Support network meetings of patient representatives who are participating in HTA or may do so in future
	Building capacity	Patient groups	Promote trusted online training resources
	Building capacity	Patient group members	Support attendance at HTA conferences with travel grant
	Building capacity	Individual patients	Payment for travel, loss of earnings, preparation of submission
		Patient groups	
	Building capacity	Patient groups	Organize a buddying system among patient representatives
	Legitimacy	Patient groups	Include patient representatives in conference organization committees
	Fairness Relevance	Patient groups	Offer grants for projects related to HTA
Individual HTAs			

(continued)

Table 5.3 (continued)

Phase (stage)	Why (goals/values)	Who to involve	How—mechanisms that HTA body could organize
Scientific advice on study design	Relevance	Individual patients	Patients considered equal expert in scientific advice meeting
	Relevance	Individual patients	Meeting led by HTA researcher to elicit issues to feed into scientific advice meeting
HTA scoping/protocol development	Relevance	Individual patients	Interviews/focus groups to identify key issues to hone research questions and identify priority patient populations (e.g. with high unmet need)
	Relevance	Patient groups	Stakeholder consultation on draft scope/protocol and PICO[b] framework
	Relevance Equity Fairness	Individual patients	Workshop to discuss PICO[b] framework
Primary or secondary research	Fairness Equity	Patient groups	Input to design, conduct, and reporting of research as per public research guidance[c]
Submission of patient input	Fairness Relevance	Patient groups	Via clear template with supporting guidance and assistance from HTA staff
		Individual patients	
Consultation on draft report	Relevance	Patient groups	Stakeholder review of draft reports to ensure all relevant patient issues have been included
	Equity	Patient groups	Comment on clarity of draft recommendations
	Fairness	Disadvantaged patients	Meetings to discuss draft findings
Multi-stakeholder review/appraisal of evidence/development of recommendations	Fairness Equity Legitimacy	Individual patients	Expert testimony to appraisal committee
	Fairness Equity Legitimacy	Patient groups	Comments on issues that might have been misunderstood and discusses value of treatment to patients
	Relevance Legitimacy	Patient groups	Separate section of HTA report summarizes patient aspects
	Legitimacy Relevance	Patient groups	Summaries of patient input
		Public representative	
Appeal	Fairness Legitimacy	Patient groups	As a stakeholder according to defined appeal process, or to the courts
Communication	Relevance	Patient groups	Input to development of patient friendly summaries of HTA[d]
	Legitimacy	Patient groups	Participation in press conference about HTA decision
		Individual patients	
	Legitimacy	Patient groups	Dissemination of HTA recommendation
	Relevance	Patient group	Share patient group submission with umbrella patient organizations
Managed entry to health service	Equity Legitimacy	Patient groups	Act as 'safe harbour' to provide governance on data collection systems for managed entry agreements

[a]Includes informal caregivers/carers
[b]Population, intervention, comparator, outcomes
[c]INVOLVE (2012), PCORI (Chap. 30)
[d]See the work of DECIDE (2015)

have to gather or contribute specific information to the HTA, either in writing or verbally. Issues related to patient input are explored further in Chap. 6.

The quality of the participation process relies on the patient or patient representative's ability to contribute competently. So there is a need to build capacity with patients and patient groups about HTA processes and agree how they can best contribute. This was an important point raised in the development of the HTAi Values and Quality Standards for Patient Involvement in HTA, where after the first Delphi Round, a new value of 'building capacity' was added. This value features strongly in Table 5.3, and for effective patient participation in HTA, at least some of these capacity building elements should be undertaken.

5.6 Discussion

Over the past decade, various surveys and literature reviews have shown that some HTA bodies report that patients can participate in their processes. However, this participation is often limited to standard processes created for all stakeholders (submission of topics via the web or public consultation on draft reports), with little consideration of the enablers of patient participation. For others, patient participation in HTA is still contentious (Gauvin et al. 2011) and is not encouraged.

There are many reasons why HTA bodies are reticent to support participation of patients in the HTA process, including issues such as:

- The adversarial nature and lobbying tactics that some patient organizations employ.
- Concerns that patient groups' views are biased and influenced by health technology developers.
- Concerns about lack of representativeness.
- Resource requirements to support patient participation and change HTA processes to accommodate them.
- How patient input can be balanced against scientific evidence.

On the other hand, patient groups question the value of their contributions to HTA (SECOR 2012, Dickson personal communication 2016) including concerns about:

- What contribution is required from them and how 'scientific' it needs to be.
- How their contribution is balanced against scientific evidence.
- The time and effort required to participate in a submission, which for patient groups has a real opportunity cost from their voluntary donations and granted funding sources.
- How they can contribute as 'partners' (or address potential decision uncertainties) when they are not fully involved and do not receive the same information as others that are involved.

- Being treated as novices, when they may have highly competent staff who input to a range of health policy initiatives and members who can articulately share relevant experiences.

Coulter (2004) indicated that patients need to understand the choices confronting policy makers and be involved in determining priorities and trade-offs. This can be challenging as they are often seen as having a vested interest in health technologies that relate to them (Chap. 3). So it is important that patient groups are involved in creating fair, consistent and transparent processes for HTA. This includes issues like ensuring those invited to participate are sufficiently supported and have adequate time to input and that as experiential experts, patients and their representatives are treated like other experts, including declarations of potential conflicts of interest and given appropriate feedback.

Participation is dynamic (Tritter and McCallum 2006) and should be a two-way dialogue (Rowe and Frewer 2005). So the essential element of any participation process is the dialogue to review processes and continually improve them (Chap. 16).

5.7 Conclusion

Beyond the democratic goal of participation, the most important goal of patient participation seems to be instrumental, to improve value judgements in HTA, by elucidating issues about the impacts of the technology that are missing in traditional clinical and economic evidence. This could include:

- Focussing research questions on issues that matter to patients.
- Describing the populations affected by condition, their natural history, use of current health technologies and those with greatest need.
- New insights to interpretation of clinical evidence and the patient benefits that might be achieved.
- Input to economic model structures—clinical pathways, potential consequences of technology use and utilities.
- Communicating results in a manner that is meaningful to patients.

Most HTA bodies have systematic approaches to literature review and economic modelling, so there should likewise be systematic processes for patient participation that gather input and report it clearly, demonstrating how it contributes to recommendations and decisions. The mosaic for patient participation in HTA presented here is a starting point for HTA bodies to draw from. The aim is to outline good processes for patient participation and see them promoted and developed in order to optimize the HTA process (Gauvin et al. 2010).

The mosaic presented in Table 5.3 has received some input from the country authors in Part III, but it is still in development. In particular questions have been raised about the goals column, which here refer to the HTAi values for patient involvement in HTA. When used by an HTA body, they may be more specific—for example, to find out information about the patient pathway or to understand the impact of side

effects. Likewise the 'who' column may vary and be more specific. Furthermore, the mosaic needs better presentation than the current tabular form. The important point is to note that a range of approaches is possible and to choose those that the HTA body and patient group advisers think are optimal and add value to the HTA process, recognizing that patient participation is bespoke to the organization, complex and evolutionary.

Although this chapter considers processes to support patient participation in HTA, it is not promoting patient participation above patient-based evidence. Indeed, given the rigour of HTA, patient-based evidence should be integral to HTA. However, given its demise in most HTAs, there seems to be a need to ensure systematic processes to include patients' perspectives in some form that is practical in rapid HTA processes. Also given the interest in patient input, it is important to ensure that other forms of participation are supported that do not place the burden of contribution too heavily on the patient or patient group.

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References

Arnstein SR. A ladder of citizen participation. J Am Inst Plann. 1969;35:216–24.

Barello S, Graffigna G, Vegni E, Bosio AC. The challenges of conceptualizing patient engagement in health care: a lexicographic literature review. J Participat Med. 2014;6:e9.

Boivin A, Lehoux P, Burgers J, Grol R. What are the key ingredients for effective public involvement in health care improvement and policy decisions? A randomized trial process evaluation. Milbank Q. 2014;92:319–50.

Bridges JFP, Jones C. Patient-based health technology assessment. Int J Technol Assess Health Care. 2007;23:30–5.

Coulter A. Perspectives on health technology assessment: response from the patient's perspective. Int J Technol Assess Health Care. 2004;20:92–6.

DECIDE. Developing and evaluating communication strategies to support informed decisions and practice based on evidence – patients and public. 2015. http://www.decide-collaboration.eu/patients-and-public Accessed 1 Nov 2016.

EPF. Patient involvement in health technology assessment in Europe – results of the EPF survey. Brussels. EPF. 2013. http://www.eu-patient.eu/globalassets/projects/hta/hta-epf-final-report2013.pdf. Accessed 6 Mar 2016.

Facey K, Hansen HP. Patient-focused HTAs. Int J Technol Assess Health Care 2011; 27:273-274. doi:10.1017/S0266462311000572.

Facey K, Stafinski T. Tools to support patient involvement in HTA. In: G-I-N public toolkit: patient and public involvement in guidelines. 2nd ed; 2015. p. 141–59.

Gagnon M-P, Desmartis M, Lepage-Savary D et al. Introducing patients' and the public's perspectives to health technology assessment: a systematic review of international experiences. Int J Technol Assess Health Care 2011; 27:31-42. doi:10.1017/S0266462310001415.

Gagnon MP, Desmartis M, Gagnon J et al. Framework for user involvement in health technology assessment at the local level: views of health managers, user representatives, and clinicians. Int J Technol Assess Health Care 2015; 31:68-77. doi:10.1017/S0266462315000070.

Gauvin F-P, Abelson J, Giacomini M, Eyles J, Lavis JN. "It all depends": conceptualizing public involvement in the context of health technology assessment agencies. Soc Sci Med. 2010;70:1518–26.

Gauvin FP, Abelson J, Giacomini M, Eyles J, Lavis JN. Moving cautiously: public involvement and the health technology assessment community. Int J Technol Assess Health Care 2011; 27:43-49. doi:10.1017/S0266462310001200.

Gauvin FP, Abelson J, Lavis JN. Evidence brief: strengthening public and patient engagement in health technology assessment in Ontario. Hamilton, Canada: McMaster Health Forum; 2015.

Hailey D. Consumer involvement in HTA. Alberta: Alberta Heritage Foundation for Medical Research HTA Unit, 2005. http://www.ihe.ca/download/consumer_involvement_in_health_technology_assessment.pdf. Accessed 2 Feb 2016.

Hailey D, Werko S, Bakri R, et al. Involvement of consumers in health technology assessment activities by INAHTA agencies. Int J Technol Assess Health Care. 2012;29:79–83.

IAP2. IAP2's public participation spectrum. 2016. http://c.ymcdn.com/sites/www.iap2.org/resource/resmgr/Foundations_Course/IAP2_P2_Spectrum.pdf?hhSearchTerms=%22spectrum%22. Accessed 19 Aug 2016.

IJTAHC. Int J Technol Assess Health Care. 1998;14(1)

IJTAHC. Int J Technol Assess Health Care. 2011;27(4)

INVOLVE. Briefing notes for researchers: involving the public in NHS, public health and social care research. Eastleigh: INVOLVE; 2012. http://www.invo.org.uk/wp-content/uploads/2012/04/INVOLVEBriefingNotesApr2012.pdf. Accessed 1 Nov 2016

Kreis J, Schmidt H. Public engagement in health technology assessment and coverage decisions: a study of experience in France, Germany and the United Kingdom. J Health Polit Policy Law. 2013;38:89–122.

Maxwell GL. The state of advocacy in cancer. Gynecol Oncol. 2015;139:573–9.

Menon D, Stafinski T. Role of patient and public participation in health technology assessment and coverage decisions. Expert Rev Pharmacoeconomics Outcomes Res. 2011;11:75–89.

Menon D, Stafinski T, Dunn A, Short H. Involving patients in reducing decision uncertainties around orphan and ultra-orphan drugs: a rare opportunity? Patient 2015: 8:29–39. doi: 10.1007/s40271-014-0106-8.

OHTAC Public Engagement Subcommittee. Public engagement for health technology assessment at health quality Ontario–final report from the Ontario health technology advisory committee public engagement subcommittee [internet]. Toronto: Queen's Printer for Ontario; 2015.

Rowe G, Frewer L. A typology of public engagement mechanisms. Sci Technol Hum Values. 2005;30:251–90.

Royles D. AIDS and AIDS activism. Encyclopedia of greater Philadelphia. Philadelphia: University of Pennsylvania Press; 2012. http://philadelphiaencyclopedia.org/archive/aids-and-aids-activism/

SECOR. CADTH patient input process review – findings and recommendations. 2012. https://www.cadth.ca/media/pdf/2012_SECOR_Patient-Input-Review_e.pdf. Accessed 8 Mar 2016.

Tritter JQ. Revolution or evolution: the challenges of conceptualizing patients and public involvement in a consumerist world. Health Expect. 2009; 12:275–287. doi:10.1111/j.1369-7625.2009.00564.x.

Tritter JQ, McCallum A. The snakes and ladders of user involvement: moving beyond Arnstein. Health Policy. 2006;76:156–68.

Whitty JA. An international survey of public engagement practices in health technology assessment organisations. Value Health. 2013;16:155–63.

Chapter 6
Patient Input to HTA

Karen M. Facey and Ann N.V. Single

6.1 Introduction

The term patient input can be used to describe the information provided by patients, their caregivers and patient groups to any deliberative process that includes a range of stakeholders. Whether written or verbal, patient input aims to contribute the unique knowledge borne from the experiences of living with a condition. While patient-based evidence relies on trained researchers to study patient issues in a rigorous manner, the onus for patient input lies on patients and patient organisations gathering information and presenting it in a way to aid the multi-stakeholder discussions that occur in HTA. Patient input is complementary to research and is not generally critically assessed in the same way as scientific evidence but can aid the value judgements and decisions made at any point in the HTA process from topic selection through to communication of results. This chapter outlines a variety of patient input approaches: providing written comments on draft documents, submitting written information about specific issues via a structured form and face-to-face participation in a committee or advisory group. It reflects on processes, the burden placed on patients and patient groups, their need for support and concerns about impact of input.

The terminology of 'patient input' is adopted from the Canadian Agency for Drugs and Technologies in Health (CADTH), but it is not universally accepted. Patient input can inform colloquial evidence (Sect. 6.4.1) alongside input of expert clinical reviewers, providing important contextual knowledge. This could be used at

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various points in the HTA process to provide insights from patients gained in the 'real world' and focus deliberations towards the implications for patients (Staley and Doherty 2016). However, patient input is unlike other expert input. It is derived from the personal, often sensitive and sometimes deeply painful, experiences of individuals. It comes to HTA not via an established and mediated scientific process, but from a process of human interaction among patients, patient groups, HTA staff and other experts that may occur before, during and after an HTA. As such, it is subject to a variety of challenges.

As patient input puts the burden of preparation of information on patients and patient groups, it is essential to clarify how it can influence an HTA. Some HTA appraisal committee members feel that as HTA is grounded in research, it cannot include informal or anecdotal information (Lopes et al. 2015, SECOR 2012). However, patient input can inform the design of clinical research (Chap. 8) and economic models and facilitate interpretation of results arising from such research, e.g. contextualising it within local healthcare settings or highlighting important outcomes not described in the published literature (see country examples in Part III). For example, 'the health technology creates such bad diarrhoea that it stops people going to work' or 'my mobility has improved so much I can now dress myself'. Such insights into patient benefit can influence an HTA, particularly when views of a diverse group of patients are presented coherently (HTAi 2015a) and when there are uncertainties about clinical benefits (Menon et al. 2015). As a past appraisal committee chair indicated in Australia, patient groups 'present data that contribute to the 'value construct' of a decision' (Lopes et al. 2015 [336]), i.e. shaping the questions we ask about using a health technology and defining what matters most.

As the concept of patient input is new, this chapter relies on the experience of the authors and their associations with HTA bodies and patient groups internationally. We argue that patient input can play an equally important but different role to patient-based evidence. It can achieve the shared learning and problem solving that occur in effective participation processes (Tritter 2009, Chap. 5), but this does not come without costs and challenges. We acknowledge that our conceptualisation may not be accepted by all, so this chapter provides a basis for further discussion and research.

6.2 Written Comments on Draft Documents

6.2.1 Topic Scoping

HTAs are often scoped using the PICO framework (Chap. 1). Comments from a patient group on an HTA scope can be a valuable way of ensuring HTAs consider the outcomes important to patients (Oliver et al. 2009, Berglas et al. 2016). The National Institute for Health and Care Excellence (NICE) publishes its draft scoping documents, all comments it receives and the final HTA scope. Reviewing any of

these can show the influence of patient input. For example, a comparison of these documents in an HTA of a treatment for relapsing-remitting multiple sclerosis shows that the patient group input influenced the scope in terms of identifying patient sub-groups and appropriate comparators (NICE 2014).

6.2.2 Consultation on Draft Reports

For some HTA bodies, the only mechanism for patient input is via public consultation on a draft HTA report. Public consultation may be targeted so that patient groups are notified of the consultation (Cowl et al. 2015) and responses may be requested in free form or by response to specific questions. At NICE, patient groups are encouraged to comment on the extent to which the draft recommendations in an HTA have taken account of patients' perspectives (Chap. 23).

However, HTA reports can be large scientific documents, which are difficult for patient groups (and health professionals) to comment on. Short consultation periods exacerbate the problem, especially if patient groups want to canvass the views of their members. The problem can be alleviated with short, plain language adaptations of consultation documents and workshops during consultation periods to present the report to patients and help them consider the issues in question before they submit their response (Cowl et al. 2015).

6.3 Submitting Written Information

6.3.1 Topic Proposals

Some HTA bodies (such as SBU and the Scottish Health Technologies Group (SHTG)) accept topic proposals from a range of stakeholders, including patient groups. However, completing the complex topic proposal forms can be challenging for all stakeholders, even when assistance from HTA researchers is offered.

6.3.2 Submissions to Appraisal Committees/Multi-stakeholder Advisory Groups

Many HTAs have moved from comprehensive systematic reviews of published literature to structured submissions of evidence from health technology developers (Chap. 1). This development raised the possibility of patient groups making submissions in a similarly structured way. Some HTA bodies (e.g. Scottish Medicines Consortium (SMC), NICE and CADTH) encourage written submissions from patient groups, whereas others (e.g. Pharmaceutical Benefits Advisory Committee

(PBAC) in Australia, National Committee for Health Technology Incorporation (CONITEC) in Brazil, National Health Insurance Administration (NHIA) in Taiwan) have an open call via their website to anyone, including individual patients.

The HTA bodies in the UK and Canada were the first to implement standardised templates to capture patient group input that included prompts about their experiences and expectations of new and existing medicines (Chap. 21, Chap. 23, Chap. 27).

To share good practice, the HTAi Interest Group for Patient and Citizen Involvement in HTA (the HTAi Interest Group) published a *Patient Group Submission Template for HTA of Medicines* (HTAi 2014). The template was developed from an amalgamation of existing templates and consultation in HTAi. After this, HTAi developed the *Patient Group Submission Template for HTA of Health Interventions (not-medicines)* (HTAi 2015b). During consultation on this template, it was recognised that a new template for diagnostic technologies should be developed and it will be published in 2017.

These submission templates include a section to capture descriptive information about the patient organisation. They provide guidance about the form of information that may be most influential for an HTA committee, noting that the following issues should be explained:

- Sources—individual patient stories, review of patient group helpline queries, surveys, social media, workshops, documentation of clinic visits, etc.
- Strength—how many patients and methods for each source
- Breadth—representativeness of statements compared with the views of the many patients that might be treated with this technology, including those that are seldom heard.

The templates ask a few main questions, with prompts to indicate the information that is most likely to influence an HTA committee or advisory group. Key elements are summarised below.

HTAi Patient Group Submission Templates: Summary of Key Elements
(Adapted from HTAi 2015b)

Impact of the condition

- Challenging symptoms
- Limitations to usual activities of daily living
- Emotional and psychological issues
- Impacts on family life
- Financial implications
- Impacts on caregivers

- Groups of patients that have particular issues
- Impact on caregivers

Experiences with currently available health interventions

- Main health interventions currently used
- Extent to which they control/reduce the most difficult aspects of the condition
- Most important benefits of currently available interventions
- Burden of taking currently available health interventions on daily life
- Side effects that are distressing or difficult to tolerate
- Financial implications to the patient and his/her family
- Implications for caregivers
- Areas the current health interventions do not address
- Groups of patients that have particular issues with current interventions

Experiences with the health intervention being assessed (views from patients who have received the technology)

- Main reasons for use of this health intervention compared with other interventions
- Extent to which the health intervention controls or reduces the most difficult aspects of the condition (symptoms, daily life, quality of life, fewer hospital visits)
- Limitations of the health intervention
- Side effects that are difficult to tolerate and those that patients are willing to tolerate
- The burden of taking the health intervention on daily life
- Financial implications to patients and families
- Impact of intervention on caregivers
- Aspects of the health intervention that patients would like to change

Expectations of the health intervention being assessed (views from patients who have not received the technology)

- Whether the studied outcomes are important to patients
- Minimum level of improvement of the most important symptoms that patients would like to see
- What patients would most like to see from the intervention being assessed
- Main reasons why the health intervention being assessed may not be used
- Perceived advantages and disadvantages
- Financial implications to patients and their families
- Impact of health intervention on caregivers
- Groups of patients that might benefit most from the health intervention

The templates have a cover note to help HTA bodies adapt them for their own processes (HTAi 2016b). In France, the Haute Autorité Santé has translated and adapted the templates to suit their HTA appraisal process (Nabarette and Guerrier 2016), while the UK, NICE and SMC have used them to inform updates of their own templates. Countries such as Sweden, Taiwan, Finland and Australia have translated and adapted/or piloted the HTAi patient group submission templates.

6.3.3 Reflections on Submissions of Written Information

While the adoption of a standardised template to capture patient issues may appear relatively simple and especially useful for the timelines of rapid HTAs, we do not view it as a substitute for patient-based evidence nor a quick solution to patient participation. The use of written submissions creates challenges for patient groups and HTA bodies; some of which are discussed below.

As it takes time and specific skills to complete submission templates, many patient groups require information about how to collect and report this information. For example, the pan-Canadian Oncology Drug Review (2013) has published guidance on undertaking surveys and interviews and presenting the results, and this has been adapted to suit the HTAi submission template (HTAi 2015a). As these activities involve patient groups collecting information from patients, those without experience in the area may need information about ethical and legal issues to help them safeguard the dignity, rights and well-being of patients when preparing submissions to HTA (Single et al. 2016). These issues include using existing sources of information when possible; choosing appropriate methods; including a broad population; protecting data and privacy; and ensuring participants can competently consent and have sufficient information, such as how their information will be used and realistic expectations for its influence (HTAi 2016a, b).

As HTAs are increasingly performed before a health technology is made routinely available in a health system, the only opportunity for patients to have experienced a new technology is in research. The Canadian Treatment Action Council has called for CADTH to take leadership in connecting patient groups with clinical trial participants (Berglas, Personal communication, 2016). However, if a patient group cannot identify any patients who have received the new technology, this should not deter them from making a submission. The information they can provide about living with the condition and experiences of current practice is still valuable.

Some HTA bodies allow individual patients to submit comments on a structured template via a weblink, but this can result in a large number of submissions that must be summarised by an HTA researcher or a patient representative on the committee or advisory group. There are also challenges in considering how individual patients can be supported to make submissions. Furthermore, it would seem that using patient groups as intermediaries with patients is a better solution or that research should be undertaken with patients to understand their experiences, without putting the onus on individuals to formulate an input.

Some HTAs may attract several patient group submissions. Some argue that multiple submissions stressing the same issues are valuable. Others feel that one submission from several patient groups provides stronger input. Creating joint input with clinical experts could also be valuable (Chap. 27). However, in such situations it is essential to ensure that the contribution of patient groups is equal to that of clinicians and that any differences in views are documented.

A major issue for patient groups is to understand how their written submissions are used in the HTA appraisal process (SECOR 2012). In some cases, researchers may provide a written summary, in others, 'public partners' may present the patient input and the full patient submission may be available in appraisal committee papers. Public partners differ among HTA bodies, but usually they are volunteers (paid or unpaid) who have been specifically recruited and trained to help ensure patient issues are considered in HTAs.

HTA submissions are highly complex and committee members will have different expertise and several HTA products to review at one meeting. So, committees often do not take a detailed presentation of any part of the evidence, but rely on summaries provided by HTA staff, with discussion focusing on areas of uncertainty. Patients may feel their input is poorly presented or not addressed by the decision-making committee. This can be challenging for patient groups who have put great effort, time and resources into a submission. An experienced public partner can interject relevant information from a patient group submission to help resolve issues emerging in the deliberative discussion. This has pros and cons, as the public partners understand the HTA methodology and committee dynamics, but if patient groups had known the uncertainties in advance they may have submitted different input. Also, it means that the public partners must be competent and well trained to engage in the discussion. At the time of writing, the role of public partners in relation to presentation of patient issues is under debate and will no doubt evolve.

Feedback to patient groups about their submissions is essential to help them understand how their input has been taken into account and how their submissions can be improved to have more influence (Genetic Alliance UK 2014). Methods of feedback may include setting out how patient input has been taken account of in the HTA report; publishing examples of high-quality submissions on HTA websites; or providing videos sharing the experiences of those who have submitted, letters detailing the strengths and weaknesses of the submission and face-to-face meetings to discuss outcomes.

These issues highlight the importance of dialogue between patient groups and HTA bodies about the best processes for written submissions. This interaction is key and part of the journey of patient participation that builds relationships, understanding and trust.

6.4 Face-To-Face Input

Face-to-face input potentially enables patient input to occur at several points in an HTA and in a variety of settings which may be more accessible to patients and patient groups. While the strength of written submissions lies in the work undertaken to prepare them, the strength of face-to-face input is its ability to respond to

questions and clarify misunderstandings. NICE combines the two by giving patients groups the opportunity to present their input. Additionally, Germany's Joint Federal Committee (G-BA) and the Institute for Quality and Efficacy in Health Care (IQWiG) supplement written submissions with 'hearings' (Chap. 25) and PBAC recently began consumer hearings for some medicines (see Sect. 19.5) so that patient groups or representatives can answer committee members' questions.

Face-to-face input may be considered in two ways—where the patient representative is an equal member of the group or where they are invited to a group or committee to input on specific issues. Face-to-face input where patients/patient groups are an equal member of a committee is the only form of input that allows the patients to react to the discussion as it emerges, adding further information or clarifications. Two of the most common times when face-to-face input is used in HTA are outlined in this chapter.

It has been suggested that to ensure face-to-face input of patients is effective, it can be helpful to prepare patients by explaining not only the process of the meeting but also the meeting norms (seating arrangements, dress, style of presentations) and what will be expected of them (HEE 2008). Special needs of patients (access, diet, visual or audio support) need to be clarified in advance. Payments should be made for expenses incurred, and some HTA bodies pay a meeting fee according to organisational policies. Furthermore, the others involved in the meeting need to be prepared to listen to patient input and the chair (or facilitator) needs to receive specific guidance on how to encourage patient input (Thomas and Meredith 2015).

6.4.1 Scientific Advice (Early Dialogue)

Several HTA bodies have established systems to provide advice (sometimes called early dialogue) to health technology developers about the design of their confirmatory trials (CADTH 2016, NICE 2016, EUnetHTA 2016). These processes involve professionals from the HTA body and invited clinical experts. More recently, they have sought to involve patients (Facey et al. 2015), recognising that patients can contribute valuable information about important outcomes and practicability of clinical investigations proposed in the trial. In these meetings, patients contribute as individuals bringing their own experience into the discussion. However, the optimal process for patient participation has yet to be found given the very technical nature of the meetings. Some HTA bodies involve individual patients with the relevant stage of disease in the meeting (Facey et al. 2015); others run focus groups with patients in advance to address issues that can be fed into the meetings (Chap. 21).

6.4.2 Involvement in Multi-stakeholder Committees

Patients and patient representatives may be invited to contribute to a range of committees/advisory groups during an HTA (from topic selection through to appraisal). Individual patients can set the context by giving their own experience of the

condition and patient groups can share patients' experiences relating to the health technology that may fill gaps in the evidence (e.g. will patients really follow usage instructions) or provide context and impact (e.g. actual care pathway, most debilitating side effects, what a complex outcome really means for patients, long-term consequences). Given the technical nature of such meetings, it is important that patients are prepared for the meeting and invited to contribute by the chair.

6.5 Patient Input: Burden or Bonus?

6.5.1 Burden and Building Capacity

Patient input places a burden on patients and patient groups, who are often working on a voluntary basis, with few resources and experiencing ill health. Furthermore, some patient groups may be asked to provide input to several HTAs in 1 year or different HTA processes in their jurisdiction (e.g. UK groups submitting to England, Wales and Scotland). HTA bodies and patient groups could work together to reduce the burden by agreeing information that is relevant for several appraisals or that can be prepared in advance. Patient input requests could be targeted to areas of uncertainty (e.g. when the magnitude of effect on a complex clinical outcome is questioned or when a cost-effectiveness analysis is just above the willingness to pay threshold that is usually accepted). The challenge is how to identify such cases in advance.

There is an opportunity for umbrella patient groups to prepare information about living with the condition and using current health technologies that is common across countries as a resource for national groups to draw upon. This raises the potential for identifying what information is needed for each assessment and developing repositories of patient issues relating to the condition and current health technologies. An alternative approach is the FDA's new Voice of the Patient reports (FDA 2016) which places the burden of activity on the regulatory organisation, which organises a workshop and writes the report.

As Chap. 5 highlights, patient participation requires HTA bodies to build the capacity of patient groups who participate in their processes. For patient input, specific training may include:

- The purpose of HTA
- Local HTA processes
- Collecting and reporting patient information that is most likely to impact HTAs
- Presenting at committee meetings
- Communicating HTA recommendations.

The quality and number of submissions is improved when HTA bodies provide support staff (Chap. 27) and encourage patients who have been through their process to be mentors and share stories of their experiences (Genetic Alliance UK 2014). Additionally, HTA bodies can provide opportunities for networking among patient groups, conference participation, joint research and publication production.

Many HTA bodies also provide a large range of tools to support patient input, for example, the HTAi Interest Group website offers links to guides, glossaries, films and e-training for patient groups (HTAi 2016c).

6.5.2 Bonus vs Balance

At present, our understanding of the difference (bonus) that patient input makes is anecdotal. Some examples are given in the country experiences in Part III, but we need to do more to capture these examples (Chap. 16). Berglas et al. (2016) note that CADTH received 297 patient input submissions to 142 HTAs over a five-year period and describe how this input was used in the scoping, assessment and appraisal phases of the CADTH Common Drug Review HTA process. In the qualitative review of 30 of these HTAs (ibid), they identified 119 patient insights about health status, progress of recovery and sustainability of health, and note that this patient input helped frame the HTAs and interpret the other HTA evidence.

As discussed in Chap. 5, a range of mechanisms might be needed to achieve the goals of patient participation. Likewise, several forms of patient input might be needed in an individual HTA. NICE take comments on draft documents followed by face-to-face meetings to develop the scope of their HTAs. It also takes patient group submissions and invites a patient and patient group representative to its appraisal committees. SMC takes patient groups submissions to its appraisal committee and more recently has hosted a joint meeting of clinicians and patients to develop a shared patient and clinician statement for the appraisal committee.

One of the key questions currently raised about HTA is how to balance different forms of evidence (Chap. 4). HTA has always had to manage evidence from different sources. Even in systems where HTA is based on cost-effectiveness, evidence about the therapeutic context, functioning of the technology and clinical effectiveness are presented first. However, as some authors suggest, it may not be appropriate to balance patient input against evidence of clinical and cost-effectiveness (SECOR 2012). Instead, patient input, like input from clinicians and researchers, may provide a lens through which to assess or appraise the evidence that is presented in HTA.

There have been concerns that patient groups may have potential conflicts of interest, particularly if they receive funding from health technology developers (Hughes and Williams-Jones 2013). HTA bodies have tried to address these concerns by requiring patient groups to disclose interests in a variety of ways. In the CADTH oncology appraisals process, a patient group can only contribute if they receive funding from more than one health technology developer and no single health technology developer should provide more than 50% of the group's operating funds (CADTH-pCODR 2015). However, this may be a disadvantage for rare disease groups where there may be only one technology developer. SMC does not prohibit any patient group from submitting information, instead it presents the percentage of overall funding the patient group has received from the pharmaceutical industry (with specific information about whether funding was received from submitting

company). This allows committee members to form their own judgement regarding the perceived conflict (SMC 2016).

Whatever process is used for patient input, the concepts of fairness, transparency and accountability suggest it is important to summarise patient input in an HTA report and indicate its influence on the final recommendations.

6.6 Discussion

This chapter shows how HTA bodies harness patient knowledge via patient input processes, encouraging patients and patient representatives to participate as experienced-based experts (Boivin et al. 2014). Patient input may not have the scientific rigour of research, but those providing input need credibility (ability to contribute knowledge that is considered valid and relevant and will result in mutual learning and generation of new solutions) and legitimacy (to speak on behalf of people affected by health services) of participants (Boivin et al. (2014). However, it must be recognised that there are also concerns about patient input processes and their requirement for a very specialist form of patient to contribute (Chap. 3).

The barriers and enablers to patient participation in HTA (Chap. 5) are particularly relevant to patient input, as the burden of input is on the patient group and patient. This is confirmed by a study in Australia that notes the barriers to patient input of poor communication, lack of transparency, unworkable deadlines, inadequate representativeness and timing of input (Lopes et al. 2015).

To continuously improve patient input practices, we need to find ways to evaluate the difference it makes, taking feedback from all those involved: patients, patient groups, HTA staff and HTA advisory groups and committees. Furthermore, HTA bodies need to collaborate with one another and other bodies (such as regulators and health technology developers) to ensure information about patients' experiences is shared and that patient input is focussed on issues that will really make a difference to an individual HTA.

Patient input is not intended to substitute research into patient aspects, which is likely to place much less burden on patients and does not require the creation of expert patients who understand HTA. Instead, patient input is a mechanism of participation that provides opportunities for constructive dialogue and shared learning between all those involved in HTA, to help HTA bridge the gap between evidence and decision-making.

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References

Berglas S, Jutai L, MacKean G, Weeks L. Patients' perspectives can be integrated in health technology assessments: an exploratory analysis of CADTH common drug review. Res Involv Engagem. 2016;2:21. doi:10.1186/s40900-016-0036-9.

Boivin A, Lehoux P, Burgers J, Grol R. What are the key ingredients for effective public involvement in health care improvement and policy decisions? A randomized trial process evaluation. Milbank Q. 2014;92:319–50.

CADTH-pCODR. Pan-Canadian oncology drug review: patient engagement guide. 2015. https://www.cadth.ca/sites/default/files/pcodr/pCODR's%20Drug%20Review%20Process/pcodr-patient-engagement-guide.pdf. Accessed 15 Nov 2016.

CADTH. Scientific advice. 2016. https://www.cadth.ca/scientific-advice. Accessed 13 June 2016.

Cowl J, Tyrrell H, Sakala C, Gracia J, Huang N. How to conduct public and targeted consultation. In: G-I-N public working group. G-I-N public toolkit: patient and public involvement in guidelines; 2015. p. 17–39. http://www.g-i-n.net/document-store/working-groups-documents/g-i-n-public/toolkit/toolkit-2015. Accessed 13 June 2016.

EUnetHTA. Evidence generation – on the road to standardisation along the life-cycle of health technology. 2016. http://www.eunethta.eu/news/evidence-generation-road-standardisation-along-life-cycle-health-technology. Accessed 13 June 2016.

Facey K, Granados A, Meyer F, Moseley J, Avetisyan R, Houÿez F. Multi-stakeholder collaboration in generating the best possible knowledge – the SEED experience. Report of HTAi 2015 panel session. Canada: Health Technology Assessment International; 2015. http://www.htai.org/fileadmin/HTAi_Files/ISG/RegulatoryInteractions/HTAi_SEED_Panel_Report_final.pdf. Accessed 23 Aug 2016

FDA. The voice of the patient: a series of reports from FDA's patient-focused drug development initiative. 2016. http://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm368342.htm. Accessed 22 Aug 2016.

Genetic Alliance UK. Patient perspectives and priorities on NICE's evaluation of highly specialised technologies – patient charter. 2014. https://www.geneticalliance.org.uk/media/1850/hst-patient-charter_final.pdf. Accessed 27 Aug 2016.

Health Equality Europe. Understanding health technology assessment (HTA). 2008. http://www.htai.org/fileadmin/HTAi_Files/ISG/PatientInvolvement/v2_files/Resource/PCISG-Resource-HEE_ENGLISH_PatientGuidetoHTA_Jun14.pdf. Accessed 13 June 2016

HTAi. Patient group submission template for HTA of medicines. 2014. http://www.htai.org/fileadmin/HTAi_Files/ISG/PatientInvolvement/v2_files/Resource/PCISG-Resource-HTAi_Patient_Submission_Template_v1_30-May14.doc. Accessed 22 Aug 2016.

HTAi Patient and Citizen Involvement Interest Group. Completing a patient group submission template: guidance for patient organisations for health technology assessments and appraisal of medicines. 2015a. http://www.htai.org/fileadmin/HTAi_Files/ISG/PatientInvolvement/v2_files/Resource/PCISG-Resource-GuidanceandChecklist-Dec14.pdf. Accessed 7 June 2016.

HTAi. Patient group submission template for HTA of health interventions (not medicines). 2015a. http://www.htai.org/fileadmin/HTAi_Files/ISG/PatientInvolvement/v2_files/Resource/PCISG-Resource-NonMeds_HTAi_Patient_Submission_Template_v1_Jun16.doc. Accessed 13 June 2016.

HTAi. Patient group submission template for HTA of health interventions (not medicines) – overview. Instructions for HTA organizations. 2015b. http://www.htai.org/fileadmin/HTAi_Files/ISG/PatientInvolvement/v2_files/Resource/PCISG-Resource-NonMeds_HTAi_Patient_Submission_Template_COVER_v1_Jun16.doc. Accessed 22 Aug 2016.

HTAi Patient and Citizen Involvement Interest Group. Key ethical considerations for patient groups collecting and reporting information for HTA submissions: short guide. 2016a. http://www.htai.org/fileadmin/HTAi_Files/ISG/PatientInvolvement/v2_files/Resource/PCISG-Resource-EthicsShortGuide_May16.pdf. Accessed 28 May 2016.

HTAi Patient and Citizen Involvement Interest Group. Key ethical considerations for patient groups collecting and reporting information for HTA submissions: long guide. 2016b. http://www.htai.org/fileadmin/HTAi_Files/ISG/PatientInvolvement/v2_files/Resource/PCISG-Resource-EthicsLongGuide_May16.pdf. Accessed 28 May 2016.

HTAi. Health technology assessment international–for patients and patient groups. 2016c. http://www.htai.org/interest-groups/patient-and-citizen-involvement/resources/for-patients-and-patient-groups.html. Accessed 7 June 2016.

Hughes D, Williams-Jones B. Coalition Priorité cancer and the pharmaceutical industry in Quebec: conflicts of interest in the reimbursement of expensive cancer drugs? Healthcare Policy. 2013;9:52–64.

Lopes E, Street J, Carter D, Merlin T. Involving patients in health technology funding decisions: stakeholder perspectives on processes used in Australia. Health Expect. 2015;19:331–44. doi:10.1111/hex12356.

Menon D, Stafinski T, Dunn A, Wong-Rieger D. Developing a patient-directed policy framework for managing orphan and ultra-orphan drugs throughout their lifecycle. Patient. 2015;8:103–17. doi:10.1007/s40271-014-0108-6.

NICE Technology Appraisal Guidance [TA 320]. Dimethyl fumarate for treating relapsing-remitting multiple sclerosis. 2014. www.nice.org.uk/guidance/ta320/history. Accessed 13 June 2016.

NICE. Scientific advice. 2016. https://www.nice.org.uk/about/what-we-do/scientific-advice. Accessed 13 June 2016.

Nabarette H, Guerrier M. How to develop patient perspective in our rapid HTA process at the French National Agency for Health? Poster at HTAi annual meeting, Tokyo, Japan, 10–14 May 2016.

Oliver S, Armes DG, Gyte G. Public involvement in setting a national research agenda: a mixed methods evaluation. Patient. 2009;23:179–90.

pan-Canadian Oncology Drug Review. Guide for patient advocacy groups: how to provide patient and caregiver input for a pCODR drug review. 2013. http://www.htai.org/fileadmin/HTAi_Files/ISG/PatientInvolvement/v2_files/Resource/PCISG-Resource-pCODR_Patient_Guide_May13.pdf. Accessed 31 Aug 2016.

SECOR. CADTH patient input process review – findings and recommendations. 2012. https://www.cadth.ca/sites/default/files/pdf/2012_SECOR_Patient-Input-Review_e.pdf. Accessed 13 June 2016.

Single ANV, Scott AM, Wale J. Developing guidance on ethics for patient groups collecting and reporting patient information for health technology assessments. Patient. 2016;9:1–4. doi:10.1007/s40271-015-0143-y.

Scottish Medicines Consortium. Patient group registration form. 2016. https://www.scottishmedicines.org.uk/Public_Involvement/PGP_registration_form.docx. Accessed 15 Nov 2016.

Staley K, Doherty C. It's not evidence, it's insight: bringing patients' perspectives into health technology appraisal at NICE. Res Involv Engagem. 2016;2:4.

Tritter JQ. Revolution or evolution: the challenges of conceptualizing patient and public involvement in a consumerist world. Health Expect. 2009;12(3):275–87. doi:10.1111/j.1369-7625.2009.00564.x.

Thomas V, Meredith B. The role of the chair in patient and public involvement: training and support. In: G-I-N public working group. G-I-N public toolkit: patient and public involvement in guidelines; 2015. p. n64–75. http://www.g-i-n.net/document-store/working-groups-documents/g-i-n-public/toolkit/toolkit-2015. Accessed 13 June 2016.

Chapter 7
Discussion: Attending to Values and Quality of Patient Involvement in HTA

Vikki A. Entwistle and Stacy M. Carter

7.1 Introduction

This chapter discusses Part I of this book, which has provided an introduction to the history and development of HTA and to the various rationales for, and forms of, patient involvement within it. One of the points that recurred in the introductory chapters was the sense that patient involvement in HTA is 'still contentious' to some people (Sect. 5.4), and in part because of concerns such as 'scientific debate gets softened by inclusion of patients' perspectives' (Table 5.1), and patient involvement is 'a potential hazard in achieving independent evidence-based decisions' (Sect. 3.1). To address these concerns effectively, we suggest it will be important both (1) to identify, challenge and avoid perpetuating any unfounded assumptions or faulty reasoning that might lie behind them and (2) to ensure that advocates of patient involvement in HTA avoid overstating its merits, acknowledge variability in its quality and promote the most robust and defensible approaches. In what follows, we offer a few suggestions to support both kinds of endeavour. We do this in the interests of ensuring that, moving forward, patient involvement is discussed and developed in ways that not only fulfil its advocates' aspirations that it will improve HTA but that can also convince those who are currently sceptical about its value. Our comments apply primarily to patient involvement in assessments of particular health technologies rather than in HTA policy or the development of HTA processes more generally.

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Recognising that ‘patient involvement’ and associated terms are used to refer to various things and in confusingly inconsistent ways, we follow the editors’ stipulation that in this book, ‘patient involvement in HTA’ will be used as an umbrella term for two main types of activity: the use of ‘patient-based evidence’ and more direct ‘patient participation’ in HTA processes.

7.2 Tackling and Avoiding Overgeneralisation

Perhaps the most obvious form of faulty reasoning about patient involvement to be tackled and avoided is overgeneralisation. It is clear from the chapters in Part I that people with diverse and multiple experiences and social positions can be counted among the patients who might be involved (Chap. 3), that there are diverse forms of ‘patient-based evidence’ that can be introduced into HTA in diverse ways (Chap. 4, Parts II and III) and that diverse forms of more direct patient participation, including various kinds of ‘patient input’, can be used across a range of HTA activities (Chaps. 5 and 6 and Part III). These all have a number of context-sensitive strengths and weaknesses, so generalisations about patient involvement can very quickly become inappropriate.

The contingency of some experiences with and ‘impacts’ of patient involvement also needs to be recognised. Critics should not be able to use particular historical examples, for example, of some patient groups being unwilling to consider changing their views in the light of research evidence about the effects of a technology or of patients’ perspectives having contributed to particular decisions that subsequently had problematic implications (Chap. 3), to jump with faulty inductive reasoning to the conclusions that all examples of patient involvement will have such problems. But similarly, advocates of patient involvement should not rely on examples in which particular forms of patient involvement have contributed highly distinctive and useful insights to an HTA to imply that such positive contributions will be so clearly evident whenever those (or other) forms of patient involvement are used.

7.3 Recognising HTA as Inherently Value-Laden Even in the Absence of Patient Involvement

The concern that patient involvement is a hazard in the pursuit of good HTA seems to rest to some extent on the idea that without patient involvement, HTA would be a value-free scientific endeavour—or an endeavour in which decision-makers other than patients could readily and sure-footedly adopt the right values. This kind of thinking seems to stem from utilitarian and technocratic interpretations of the basic idea that the purpose of HTA is to ensure that when questions are asked about whether and how particular technologies should be introduced into or continue to be

used within health systems, the answers are informed by systematic assessments of the health benefits, harms and costs of using those technologies. A utilitarian interpretation of this is that HTA should help ensure that health systems provide the maximum (health) benefit, with the minimum possible attendant harm, within available resources. A technocratic interpretation then assumes that these goals can be achieved using standardised methodologies such as cost-effectiveness analyses based on the results of randomised controlled trials of the technologies of interest.

However, there are several reasons to believe that these utilitarian and technocratic interpretations are insufficient. First, there are questions to be asked about what should count, and as how significant or weighty, as a '(health) benefit', 'harm' or 'cost'. Answers to these questions are, of course, value-laden. And although there are some strong areas of consensus at a general level (e.g. that reducing the burden of disease or illness is beneficial for health), the labelling and valuation of particular biomedical states and experiences as examples of disease or illness are often contested, and technologies often impact on several of these simultaneously and in different ways for different population subgroups and individuals. Evaluative research, including scientific studies of clinical and cost-effectiveness, has a normativity built into it, even if that normativity is implicit (Molewijk et al. 2003).

Secondly, other values, for example, transparency in policy decision-making, fairness in resource or benefit allocation and support for personal autonomy, can also be held to be important in society. Some stakeholders reasonably expect that HTA (in its processes and/or its outcomes) should reflect and reinforce commitments to these or other additional values—although again there is room for debate about which values, how they should be interpreted and how they should be considered in relation to each other when it is not possible to fully realise them all (Chap. 2).

Value judgements are integral to and pervasive of HTA, as has been very well argued by Hofmann and colleagues (Hofmann et al. 2014). We suggest it is further important to recognise that HTA is a value-laden enterprise *whether or not patients are involved*. Highlighting this could be one of the most important contributions and, indeed, strategies of the patient involvement movement. Once the value-laden nature of HTA is acknowledged, the field of values that is recognised as relevant can be expanded, and the need to attend to the perspectives of patients and other stakeholders (including citizens) should become more apparent.

7.4 Looking Critically at Approaches to Patient Involvement in HTA

In the inevitably value-laden process of HTA, patients are legitimate stakeholders. Writing on patient involvement should—it seems to us—start from and reflect this assumption. But this does not mean that every possible approach to or instance of patient involvement will contribute equally well as stakeholder participation, to

good HTA or to the broader fulfilment of the purposes of participation and/or HTA in society. As Hansen and Street indicated in Chap. 3, there is a clear need for humility about what patient involvement can achieve and for ongoing research and development to help improve it.

It was noted in Chap. 1 that there has been a tendency in recent years to reduce the scope and scale of assessments of particular health technologies and to rely on more direct forms of patient participation, rather than reviewing and using 'patient-based evidence'. We agree that this is worrying because it seems likely to significantly reduce the quality of patient involvement in HTA overall. Assuming a reasonable range of research studies have been conducted among people with the health conditions of interest, a careful review of these is likely to uncover a more diverse range of experiences and views than it might be reasonable to expect a few patients, or even patient advocates, to be familiar with.

A reliance on relatively direct forms of patient participation can make it particularly difficult to integrate particular patients' inevitably partial perspectives on a health condition or technology within a broader assessment of the relevant issues. The appropriateness of particular approaches to such integration will, of course, depend on who participates, under what circumstances and how. There are, however, likely to be a number of uncertainties and value tensions to be faced by HTA staff and committee members as they strive to develop a well-rounded knowledge base and appreciation of what might matter and why to key stakeholders. For example, HTA committee members will often be aware that health technology developers and others who might profit from the widespread use of a particular technology seek HTA support for its use. They know that to that end, they will often identify patients whose experiences are particularly likely to encourage a decision in favour of their technology and encourage and support them to provide input or otherwise participate in an HTA process. When these committee members hear or otherwise experience patients' input or participation, they need to bear this in mind. It would be inappropriate to dismiss these patients' accounts completely: there is a need for HTA staff and committees to try to understand what matters from patients' perspectives, and submitted accounts and comments will often be based on experiences that are deeply felt and personally important. Staff and committee members must, however, deal somehow with their recognition that participating patients are perhaps carefully selected and accounts presented to suit one set of interests and that, of course, participation can be limited in other ways. The appropriate use of patient participation is far from simple.

A recognition of the limitations of contributions made by direct forms of patient participation does not need to imply a disrespect of the people on whose accounts they are, just that an awareness that there can be constraints and other influences that tend to limit and shape what can be seen from particular positions. One combination of influence and constraint that seems particularly important at the moment is relevant to technologies to screen for and diagnose cancer. When people who live in a society with a strong culture of emphasising the importance of catching and treating cancer early experience a cancer, they are understandably likely to speak in favour of more sensitive screening and diagnostic technologies for detecting that

cancer. They cannot personally and directly recognise the overdiagnosis and overtreatment that such technologies might lead to, because these are often only evident from analyses of population level data. Thus, unless they have engaged with such analyses, these patients are unlikely to know or speak against interventions that could harm them.

The limitations of what can be seen from particular positions will also affect the insights that can be generated by research into patients' experiences, so it is important that these limitations are considered critically when that research is reviewed as well. One of the advantages of including rigorous reviews of such research within HTA processes is that critical questions, for example, about which questions the research set out to answer and how its design and execution might have influenced the completeness and robustness of its answers, can be (required to be) more explicitly considered. Reviewers (who might include or otherwise be sensitised by people with relevant experience as patients) can consider, for example, which patients were and were not included and how the ways they were recruited, observed or questioned and interpreted might have shaped what was reported about their experiences and perspectives. This form of patient involvement can be strengthened by the use and development of methodological traditions for distinguishing better quality from poorer quality work and input for all kinds of research—not just that which has tackled questions of clinical and cost-effectiveness.

7.5 Considering Talk About 'Patient-Based Evidence'

The phrase 'patient-based evidence' appears to us to have some potentially unhelpful features and implications. The appeal of adopting the term in connection with HTA is understandable as since the rise of 'evidence-based medicine' it has become rhetorically powerful to refer to 'evidence' when shoring up a decision or action. There are reasons for caution, however. The (over)use of the term 'evidence' as a near synonym for 'research', and the (over)simplistic equation of 'evidence based' with 'justified' or 'good', means the word now often fails to differentiate between what is more and less useful and so can seem rather hollow. The relationship between 'evidence about x' and 'evidence for doing y' is not always completely clear and straightforward, and the tendency for 'evidence' to be associated in health service contexts with particular kinds of 'science' also runs the risk of obscuring rather than encouraging openness about the values at play in research studies.

The distinction that is sometimes drawn between effectiveness research 'evidence' and 'patient-based evidence' is also not as clear-cut or significant as it might first seem. Good quality effectiveness research should arguably always reflect outcomes that matter to patients, and some of these will be patient reported (in which case the effectiveness research will fulfil the criteria stipulated in Chap. 4 for 'patient-based evidence').

There is also a danger of encouraging an assumption that patients will or should attach more weight to 'patient-based evidence' than to the research it is contrasted

with. We think it is important to recognise that the effectiveness and affordability or otherwise of health technologies for addressing their problems usually matter a lot to patients and perhaps especially for influencing societal decisions about the availability and use of particular technologies. Setting information about clinical and cost-effectiveness apart from [other] patient-based evidence potentially implies more of a division between patients' and others' concerns than is useful.

7.6 Concluding Remarks

We hope we have made it clear that, like other authors in this volume, we strongly endorse the need to recognise and consider how to integrate the perspectives of patients as well as those of citizens, health professionals and other experts, in important decisions about the use of health technologies in health systems and societies.

In a book about patient involvement in HTA, it makes sense to focus attention on how and how well patient involvement can contribute to HTA. However, a reorientation to a focus on the broader questions of how and how well HTA can incorporate the value concerns and insights of all legitimate stakeholders, including those of patients and citizens, might be more helpful for moving debate and action forward. Reminders that HTA is an intrinsically value-laden endeavour can bring more of the challenges of the endeavour into view. HTA requires the identification, critical analysis and defensible synthesis of a plurality of value concerns and insights relating to a potentially diverse array of relevant questions that are interlinked in complex ways. This can't be achieved by 'purely' technical means, and it might not be realistic to expect it to yield a singular universal recommendation about the use of a particular technology. Taking the perspectives of multiple stakeholders, including diverse patients, seriously may in the end require greater flexibility to be built into the recommendations made by HTA agencies. This may further complexify HTA processes, but could bring them closer to 'recognis[ing] what is best for all patients across the board' (Sect. 3.1).

References

Hofmann B, Cleemput I, Bond K, Krones T, Droste S, Sacchini D, Oortwijn W. Revealing and acknowledging value judgements in health technology assessment. Int J Technol Assess Health Care. 2014;30:579–86.

Molewijk B, Stiggelbout AM, Otten W, Dupius HM, Klevit J. Implicit normativity in evidence based medicine: a plea for integrated empirical ethics research. Health Care Anal. 2003;11:69–92.

Part II
Methodologies

Chapter 8
Patients as Collaborative Partners in Clinical Research to Inform HTA

Maarten de Wit and Laure Gossec

8.1 Introduction

This chapter introduces the concept of patient research partners (PRPs) in clinical research and presents recommendations to support PRPs that have been developed in the field of rheumatology. PRPs are encouraged to participate throughout the research process because their experiential knowledge is valued. For this collaboration to be fruitful, all participants in the research process should agree to principles of trust, respect, transparency, partnerships, communication, diversity, confidentiality and co-learning to support patient involvement in research. On this basis, recommendations are presented that relate to the role, research phases, number, recruitment, selection, support, training, acknowledgement and reporting of PRPs. This provides guidance that can help researchers and PRPs in a variety of clinical research settings such as grant assessment, agenda setting, designing and conducting a clinical study of a health technology, development of a disease-specific core outcome set including endpoints relevant to patients, patient-reported outcome measures and dissemination of findings, all of which are highly relevant to HTA processes.

National initiatives, such as INVOLVE in the UK (INVOLVE 2016), the Strategy for Patient-Oriented Research in Canada (ISPOR 2016) and the work of

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the Patient-Centered Outcomes Research Institute (PCORI, Chap. 30) in the USA, demonstrate how public funders of clinical research have been developing their processes to ensure that researchers work with patients in the design and conduct of a clinical trial. More recently, health technology manufacturers have started to explore how they can work with patients to improve the relevance and efficiency of clinical trials within the legal constraints placed upon them about interactions with patients (Chap. 33). Alongside this, there has been increasing recognition that patients must influence the design of clinical studies to ensure they meet the needs of HTA.

Case studies and systematic reviews indicate that, in the past, there has been some consultation with patients to provide input to clinical research (Shippee et al. 2015; Boote et al. 2012). Examples of more extensive collaboration with patients in particular phases of a study have also been reported, for instance, developing research agendas (Abma et al. 2014), developing research protocols (Wilson et al. 2015) or dissemination (Gagnon et al. 2009). But what do we know about patient participation throughout the research cycle? How can researchers enable patients to provide meaningful contributions to each phase of research? The recommendations presented in this chapter provide practical guidance on how patients can be included as research partners in clinical research and have relevance for both health technology developers and HTA bodies.

8.2 Patient Research Partners

Since the beginning of the century, the role of patients in clinical research has gradually become more influential. The role of passive research subject has evolved into that of patient reviewer, patient advisor and PRP. The latter role should be clearly distinguished from that of study participant who gives informed consent and enters a clinical trial to donate blood or tissue or fill out a questionnaire. Collaboration as a PRP implies equal partnership and a direct dialogue between the patient and the researcher. Here, PRPs are expected to also perform managerial and oversight roles (Dudley et al. 2015). The distinction between both roles demarcates the difference between doing research *to*, *about* or *for* patients and doing research *with* patients (Staniszewska et al. 2012).

In rheumatology, the concept of PRP was introduced by Outcome Measures in Rheumatology (OMERACT) (Hewlett et al. 2006), an international organisation that develops core outcome sets and core measurement instrument sets for clinical trials. Since 2002, PRPs have been involved in identifying new domains that are important for patients and assessing measurement instruments for content validity and feasibility (e.g. burden for patients). In 2007 the European League Against Rheumatism (EULAR) adopted the concept of PRPs to support patient-researcher partnerships in the development of disease management recommendations. At that time, patients and researchers expressed a lack of knowledge and skills on building such partnerships. This prompted EULAR to formulate a set of practical guidelines that could direct and support participants to collaborate in the context of management recommendation development (de Wit et al. 2011). More recently, also

OMERACT formalised PRP involvement by publishing practical recommendations, including a set of three overarching principles for patient involvement (Cheung et al. 2016).

OMERACT Overarching Principles for Participation of PRP

- OMERACT values the experiential knowledge of PRPs as critical to outcome research.
- Engaging PRPs as integral participants throughout the research process is a fundamental OMERACT principle.
- All OMERACT participants subscribe to the principles of trust, respect, transparency, partnerships, communication, diversity, confidentiality and co-learning with respect to patient involvement in research.

The purpose of PRPs is to provide the experiential knowledge of the impact of an illness and use of the health technology on daily life and to ensure that the perspectives of patients are preserved throughout the research process. A PRP operates as an active and equal member of the research team. A PRP can be called an expert patient when representing a patient organisation or when they are able to present a wider perspective on the disease, going beyond their individual experience. For some conditions, parents or caregivers can take on the role of a PRP. PRPs can contribute perspectives about their illness in different ways, being on a patient panel, patient reference group or guideline working group (Pittens et al. 2013) or as a member of a research steering group or Scientific Advisory Board (Teunissen et al. 2013). They can take responsibility for providing patients' perspectives in setting research priorities, research design, reviewing literature, recruitment methods, collecting data, analysis and interpretation of findings and dissemination. In addition, it is their duty to ensure that patients' perspectives are not lost at any stage of the research by providing these perspectives whenever appropriate or suggesting methods to capture these perspectives, for example, by suggesting consultation of a wider group of patients through a survey, interview, focus group or mixed method study. We believe that the added value of PRP participation outweighs potential risks and disadvantages but recognise that patients must be supported to contribute fully as partners in research.

Potential Tasks of PRPs in HTA

- Identifying questions and unmet needs that are important for patients to inform HTA agenda setting
- Promoting incorporation of patients' perspectives through existing literature or initiating new qualitative studies
- Considering ethical issues in HTA
- Critically reviewing of evidence
- Identifying and prioritising outcomes that are important for patients
- Identifying eligible target groups or subgroups

- Advocating the interests of minorities and difficult to reach groups and encouraging researchers to make additional efforts to incorporate their perspectives (e.g. through home visits)
- Demonstrating the short- and long-term real-world implications of an intervention
- Assessing the burden of treatment options in daily life
- Providing the local context of health delivery
- Advocating access to appropriate interventions
- Supporting dissemination of findings to lay audiences (for instance, by writing lay summaries or giving presentations)

8.3 EULAR Recommendations for Collaboration

The EULAR recommendations for PRP collaboration address some of the challenges of patient participation identified in Chap. 5 and provide support to researchers as well as PRPs to help avoid risks of bias and other pitfalls.

8.3.1 Role

Participation of patient research partners is strongly recommended for clinical research projects and for the development of recommendations and guidelines and should be considered for all other research projects.

How patient involvement is implemented in research depends on the objectives of a study and the health system in a particular country. Ideally, meaningful patient involvement implies combining patient contributions through various consultation methods and direct participation in the research team (such as in the example in Sect. 8.3.3). Creating opportunities for an open dialogue between patients and researchers and building sustainable relationships can be time-consuming and demanding for both PRPs and researchers. Therefore, depending on the intensity of the agreed tasks and responsibilities, a watchful eye should be kept to balance what is desirable and what is feasible.

8.3.2 Research Phases

Participation of patient research partners should be considered in all phases of the project to provide experiential knowledge, with the aim of improving the relevance, quality and validity of the research process.

In the past 10 years, PRPs have been involved in many research phases (Shippee et al. 2015). They have enriched research agendas with themes that are relevant to

patients (Abma et al. 2014) and contributed to drafting research calls, formulating research questions, developing treatment recommendations, reviewing grant applications and supporting dissemination and implementation.

Although the form and timing of involvement may be adapted to the scope of the project, it is recommended that involvement of PRPs should start as early as possible. Studies have demonstrated that involvement of PRPs in trial design frequently leads to choosing endpoints more relevant to patients, more user-friendly instruments and procedures and valuable suggestions for improving recruitment rates (Haywood et al. 2014; de Wit et al. 2013).

8.3.3 Number of PRPs

A minimum of two patient research partners should be involved in each project.

In general, it is strongly recommended to involve more than one PRP in a research project (de Wit et al. 2011; Cheung et al. 2016). This ensures multiple views from patients during meetings and continuity in the event of a relapse in illness or drop-out of one of the PRPs. It also creates opportunities for prior consultation or preparation (de Wit et al. 2011). In OMERACT it has been agreed that the research leadership takes responsibility for appropriate representation of patients' perspectives in the research project.

The primary task and responsibility of the PRPs is to help a research team think through the design and conduct of a study. Based on their personal experience with the disease and what they know about fellow patients, they may suggest phases where the perspectives of patients are relevant and advise on ways to obtain those perspectives. It is not the responsibility of the PRPs to guarantee representativeness. One or two PRPs on a research team or steering committee cannot represent *all* perspectives of the entire target population. The perspective of patients is heterogeneous as a result of age, gender, social-economic status, cultural background, disease duration and severity and other factors. Therefore the participation of PRPs should be complemented by other forms of patient involvement to enhance diversity and validity of the patients' perspectives (Legare et al. 2011). In the example below, the research team took responsibility for the integration of different forms of patient involvement in the development of the EULAR Psoriatic Arthritis Impact of Disease score (PsAID) (Gossec et al. 2013; De Wit et al. 2015a; Kirwan et al. 2016).

Example of Full Patient Participation in Clinical Research: The Development of a Patient-Reported Outcome in Psoriatic Arthritis

In the development of the PsAID, a disease-specific patient-derived quality of life instrument for psoriatic arthritis, patients were involved in various steps of the participation ladder. The involvement of patients as study participants in a domain prioritisation exercise and in the validation phase was complemented by involvement as advisors in a series of cognitive interviews in ten

countries and as collaborative partners in the overall research team. In the latter role, ten PRPs participated in two international face-to-face meetings and contributed to the:

- Identification of domains of impact important to people with psoriatic arthritis
- Definition and phrasing of items
- Translation of the draft questionnaire into the national language
- Interpretation of findings from the validation study
- Choice of recall period
- Number of the items
- Format and anchors of the instrument

All patient research partners who agreed became co-authors of the final PsAID publication.

Finally, two expert patients were member of the steering group of the project.

8.3.4 Recruitment

Identification of potential PRPs should be supported by a clear description of expected contributions.

The initiative for PRP recruitment lies with the investigator, who preferably contacts the appropriate patient organisation and provides a clear job description clarifying expectations and benefits of involvement. Not all diseases have patient organisations and only a few have established networks of trained PRPs. For this reason, some research teams have to find alternative recruitment strategies, for instance, through patient magazines, health professionals, national patient umbrella organisations or established virtual networks such as 'Patients in Research' (UK) or 'Patients Like Me' (USA). EULAR recruits PRPs through national patient member organisations, while OMERACT prefers to identify PRPs through the clinics of physician-researchers. The latter are often best situated to assess the competences of potential patients for taking on the PRP role.

All strategies involve risk of bias and have pros and cons that depend on the research context and objectives of the patient involvement. For instance, in the phase of fund-raising, the formal endorsement of a research study by a well-known patient organisation is important, while in the elaboration of a disease-specific quality of life instrument, the contributions of individual PRPs are important.

Studies have demonstrated that it is effective to discuss mutual expectations on contributions and the level of participation prior to a research project (Abma et al. 2009). This will help the researcher to create a realistic picture of the required time investment, frequency of meetings and tasks of the PRP. Conversely, the same is true for patient representatives' expectations in terms of research outcomes and

collaboration with the investigator which should be shared with the investigator. Importantly, both parties should participate in this discussion on the basis of equality and clearly specify their limits and possibilities.

As investigators and PRPs develop their thinking as a project proceeds, and needs and expectations may evolve as well, it is wise to evaluate the collaboration at regular intervals. Does the investigator provide sufficient information and support? Is the PRP not over- or underburdened and will there be a follow-up to this research project? It can also be useful for patient organisations and investigators to use the evaluation outcomes internally to adjust and improve their procedures, support and policies.

8.3.5 Selection

The selection process of patient research partners should take into account communication skills, motivation and constructive assertiveness in a team setting.

Over the years, we have learned that PRPs should not only be selected for their experiential knowledge or membership of a patient organisation but also for their competences to collaborate in a team setting. Some researchers may argue that selection for language skills, affinity or knowledge of clinical research and the ability to travel and communicate with professionals will only attract highly educated patients that are not representative for the patient group under study (van de Bovenkamp 2010). Although it is true that PRPs are not representative for the entire patient population, the fact that strict selection may indeed constitute a risk of bias is no argument to relinquish PRP involvement. Diversity of patients' perspectives should be captured through the use of appropriate research methods, and educated PRPs form an additional source for the research team (Mayer 2012).

Researchers should know that various forms of participation require various competencies and skills and should select PRPs in accordance with their expected role and tasks. Generally, the advice for researchers who start with PRP involvement for the first time is to start small and to identify two or three patients from the own institution that might be interested in clinical research. During an introduction meeting, a draft research proposal can be presented followed by an exploration among the patients for their potential interest in the study and level or intensity of involvement.

8.3.6 Support

The principal investigator must facilitate and encourage the contribution of patient research partners and consider their specific needs.

The responsibility of the investigator to enable PRPs to contribute to research in a meaningful way is crucial (Nierse et al. 2012; Hewlett et al. 2006). The investiga-

tor should provide timely and individualised information and ensure an open and safe atmosphere during meetings (Elberse et al. 2010). The type of support may vary from using understandable language and explaining difficult terms or concepts, asking open questions and inviting patients to share their perspectives to writing lay summaries, arranging access to libraries or medical databases or taking care of logistics. Depending on the role and project, the PRP may be offered a job description or formal contract, outlining issues such as responsibilities, confidentiality, conflict of interest, available support and education. Sometimes the principal investigator may appoint a designated PRP supporter in the research team. To support clinical trial teams with limited resources, the clinical trial research centre of the University of Liverpool has started to develop a toolkit that provides resources for planning, supporting, recording and evaluating patient involvement along the research pathway (Bagley et al. 2016).

A realistic budget for patient involvement and prompt reimbursement of expenses can also be regarded as support. PRP involvement requires time and money; costs for travel, accommodation, attendance fees, out-of-pocket expenses or even compensation for worked hours should be considered and subsequently realistically budgeted. Compensation for the patient association should, on occasion, be included to cover costs for recruitment, training and support. There is currently no standard for what can be considered a reasonable compensation for the work of a PRP or patient association. It depends on factors such as the PRP's preferences and the expected time investment, and it should be customised for each individual project (De Wit et al. 2016).

Network of PRPs

EULAR has established a network of over 40 educated PRPs coming from all parts of Europe and representing ten rheumatic conditions. They are involved in a broad range of research activities varying from developing disease management recommendations and reviewing grant application to participating in clinical research studies, committees of the European Medicines Agency (EMA) and Innovative Medicines Initiative (IMI) consortia and dissemination of findings. A designated coordinator at the EULAR secretariat ensures appropriate matching of research projects and PRPs and organises support and training if needed. Researchers and PRPs are provided with reference cards for collaboration and a background brochure. PRPs are invited for biannual training and evaluation meetings and alerted on training opportunities. Seven members have participated in a medicine development training course organised by the European Patients' Academy (EUPATI). In 2016 two members followed the first EULAR course on health economics in rheumatology.

In some countries, national organisations take on the task of supporting PRPs. For instance, in the UK the National Institute for Health Research (NIHR) runs induction meetings for public members who join advisory committees to help them

integrate, understand their role and meet professional members. The NIHR also provides a buddy system and organises networking events for public members to meet fellow members to share experiences and provide support.

8.3.7 Training

The principal investigator must ensure that patient research partners receive information and training appropriate to their roles.

Optimising PRP participation requires adequate capacity building. PRPs' ability to provide constructive and competent collaboration cannot always be assumed. For this reason, some institutes that foster patient participation in research offer training. Also, EUPATI offers an intensive training programme for patient representatives to understand the medicine development process (see Chap. 36). EULAR provides annual evaluation and training days to the members of the EULAR PRP network. Researchers are invited to present best practices of patient involvement or to provide additional education on, for example, critical appraisal of scientific articles or basic statistics. PRPs are encouraged to share experiences, train communication skills and learn to deal with power imbalances within a research team.

An important aim of the education is to make participants aware of the potential strengths and limitations of the role of a PRP. They learn not only to appreciate the value of personal illness and experiences of healthcare use, they also have to acquire the competence to balance their personal preferences or personal interests against other issues affecting research.

Researchers should also be taught the conditions for effective patient participation. It is often wrongly assumed that investigators have the required knowledge and competences for PRP's participation (de Wit et al. 2015b). In some countries master classes or coaching programmes are offered to familiarise investigators with the added value of patients' perspectives and methods of participatory research (de Wit et al. 2015b).

8.3.8 Acknowledgement

The contribution of patient research partners to projects should be appropriately recognised, including co-authorship when eligible.

There are many ways, both material and immaterial, to acknowledge the contribution of patients (see Table 8.1). Becoming co-authors or timely reimbursement can be regarded as examples of nonmonetary methods of acknowledgement. PRPs regularly express the lack of feedback from researchers on the value of their input and how the researcher has incorporated their comments and suggestions in the project. PRPs appreciate confirmation that their participation matters.

Table 8.1 Examples of acknowledgement

Nonfinancial acknowledgement	Financial acknowledgement
• Providing feedback on PRP input in a project or manuscript • Inviting PRPs to co-chair a meeting or to report back from a breakout session • Provide an opportunity to present patients' perspectives at a symposium or conference • Timely reimbursement of expenses • Mentioning the name of the PRP in an acknowledgement box or offering co-authorship if PRPs fulfil the International Committee of Medical Journal Editors' criteria for co-authorship • Immediate information when a grant application is approved or if a manuscript is accepted • Invitation for the Christmas event of the department • Providing a voluntary contract before the start and a certificate after finalisation of the project	• Payment of a fee or a daily allowance • Facilitate conference or symposium attendance • Making scientific information accessible; providing access to online libraries and PubMed • Birthday present or gift voucher • Providing childcare or caregiver fees • Providing a subscription to a national or international rheumatology journal

Although many patients are content with their voluntary role as PRP, the investment in terms of time and energy might become substantial and justify payment, in particular for patients who have a job and have to take days off from work to participate in a research meeting. Some organisations have rules for financial compensation for PRPs who do committee work or research projects. The resource section on the INVOLVE website (www.invo.org.uk) contains guidance and practical advice on payment and other methods for recognising the time, skills and expertise provided by PRPs. EMA has developed rules for a daily allowance, and pharmaceutical companies sometimes agree on a formal contract with PRPs paying a fixed rate per hour.

8.3.9 Reporting

The nature of PRP involvement should be reported throughout the research process, at least in the initial research proposal and final reports.

OMERACT encourages researchers to be explicit about the strategy of patient involvement. The publication policy, including peer-reviewed scientific publications and lay summaries and articles targeting the general public, should be discussed with PRPs during team meetings.

In accordance with the Guidance for Reporting Involvement of Patients and Public (Staniszewska et al. 2011), investigators are expected to report the intended or implemented patient involvement in research proposals and scientific publications, both positive outcomes and any negative consequences. Requiring reporting of patient involvement may avoid tokenism and enhance transparency of the research. It will also stimulate mutual sharing of lessons learned, challenges and

pitfalls and, by doing so, improve the systematic evaluation of patient involvement in practice. Finally, funders of clinical research want to be informed about the added value of patient and PRP involvement to legitimise their investments in this field.

8.4 Challenges

8.4.1 PRP Training and the Risk of Pseudo-professionalisation

Chapter 3 raises concerns about the professionalism of patients and their role in research. However, with the training opportunities offered by public research networks for patients seeking to become involved in research, it looks like the debate about the potential risk of patients losing their experiential knowledge as a consequence of being educated has come to an end in favour of the benefits of proper education of PRPs. However, there is still the risk that patients become professional researchers aligning easily with 'real' researchers (Dudley et al. 2015). More robust knowledge is needed to examine what kind of attitude, knowledge and skills training is needed to become an effective PRP with maintenance of the unique value of the authentic patient's experiences.

Not only PRPs need training but also researchers have to learn the basic principles and accept the practical implications of PRP involvement in clinical research. This is necessary because researchers are still reluctant to involve patients as collaborating partners. Partnership implies that control of some parts of the study should be shared with PRPs and that flexibility is required, for instance, to include new outcome measures that are important to patients although less frequently used in clinical research. It might also mean that research questions need reformulation, that inclusion or exclusion criteria need to be changed or that ways of administration or burdensome research protocols need to be adjusted to make them more patient friendly. In this regard, the King's Fund initiative has started a shared leadership programme that explores training and development interventions to establish collaborative relationships among professionals, patients and caregivers (Seale 2016).

Researchers may experience a lack of know-how or feel insecure about the amount of freedom that payers or regulators allow them to address issues important to patients. It is true that involving PRPs in the research process is time-consuming and may cause new dilemmas. Both researchers and PRPs should become aware of the practical, moral and legal implications of participatory research. Participants should also be taught the difference between the patient-health professional relationship in the clinic and that of collaborating colleagues in the context of research (Hewlett et al. 2006). This equal relationship is an essential condition for the establishment of a genuine dialogue free of power or status differences.

8.4.2 *Ethical Considerations*

With regard to the legal framework of PRP involvement, we do not have much experience in the context of clinical research. Because PRPs are not approached as *study participants* or *respondents*, uncertainty exists whether a researcher needs ethical approval to include a PRP in the research team. Similarly, although the PRP will not be exposed to any intervention, does the PRP have to sign a consent form? INVOLVE, in collaboration with the National Research Ethics Services, developed a document, stating that people do not need ethical approval when they act as specialist advisers, meaning actively involved in planning or advising on research. However, when a patient's involvement results in direct contact with study participants, the ethics committee should be consulted (www.nres.npsa.nhs.uk). This could be the case when PRPs take part in the conduct of research, for instance, by co-moderating a focus group, coding interview transcripts or assisting in recruiting by providing information to patients as a contact person. The principal investigator should ensure that PRPs are formally certified or receive appropriate support and supervision. Finally, does the PRP have to fulfil all the ICMJE[1] requirements (ICMJE 2015) to qualify for authorship of a peer-reviewed manuscript? In the absence of a standardised approach, researchers are expected to make a fair choice between an acknowledgement box or offering co-authorship.

We do not have experience of privately funded research undertaken by health technology developers, but we feel that the recommendations presented here to support inclusion of patients as research partners would inform the growing interest in 'patient-focused drug development' as outlined in Chap. 33.

8.5 Conclusion

The EULAR recommendations can help researchers to involve patients in the conduct of research, including development of successful recruitment strategies, identification of patient relevant outcomes and dissemination of results. PRP involvement requires an investment in time and commitment from the researcher. Regular exchange of mutual expectations between PRPs and researchers is beneficial and will prevent tokenism. Ensuring the representativeness of patients' perspectives and in particular the role and added value of PRPs is still challenging. It is one of the responsibilities of PRPs to help the research team to preserve patients' perspectives throughout the different stages of the research process. They are not on the team to guarantee the representativeness of the study in person because two or three PRPs can never represent the perspectives of the entire patient population. That perspective should be obtained through appropriate methods such as literature reviews, patient surveys or qualitative research, for instance, narrative

[1] International Committee of Medical Journal Editors

research, focus group or interview studies. PRPs can help researchers to improve their recruitment strategy, explore the best endpoints for a trial or ask the right questions in a focus group or survey in the right order. Future experiences will teach us how collaborations between researchers and PRPs can be fruitful in the research considered in HTA.

References

Abma TA, Nierse C, Widdershoven G. Patients as partners in responsive research: methodological notions for collaborations in mixed research teams. Qual Health Res. 2009;19:401–15. doi:10.1177/1049732309331869.

Abma TA, Pittens CA, Visse M, Elberse JE, Broerse JE. Patient involvement in research programming and implementation: a responsive evaluation of the dialogue model for research agenda setting. Health Expect. 2014; doi:10.1111/hex.12213.

Bagley H, Short H, Larman NL, Hickey HR, Gamble CL, Woolfall K, Young B, Williamson PR. A patient and public involvement (PPI) toolkit for meaningful and flexible involvement in clinical trials – a work in progress. Res Involv Engag. 2016;2:1–14. doi:10.1186/s40900-016-0029-8.

Boote J, Baird W, Sutton A. Involving the public in systematic reviews: a narrative review of organizational approaches and eight case examples. J Comp Eff Res. 2012;1:409–20. doi:10.2217/cer.12.46.

Cheung PP, de Wit M, Bingham 3rd CO, Kirwan JR, Leong A, March LM, et al. Recommendations for the involvement of patient research partners (PRP) in OMERACT working groups. A report from the OMERACT 2014 working group on PRP. J Rheumatol. 2016;43:187–93. doi:10.3899/jrheum.141011.

de Wit M, Abma T, Koelewijn-van Loon M, Collins S, Kirwan J. Involving patient research partners has a significant impact on outcomes research: a responsive evaluation of the international OMERACT conferences. BMJ Open. 2013;3 doi:10.1136/bmjopen-2012-002241.

de Wit MP, Berlo SE, Aanerud GJ, Aletaha D, Bijlsma JW, Croucher L, Da Silva JA, Glusing B, Gossec L, Hewlett S, Jongkees M, Magnusson D, Scholte-Voshaar M, Richards P, Ziegler C, Abma TA. European league against rheumatism recommendations for the inclusion of patient representatives in scientific projects. Ann Rheum Dis. 2011;70:722–6. doi:10.1136/ard.2010.135129.

de Wit M, Bloemkolk D, Teunissen T, Van Rensen A. Voorwaarden voor succesvolle betrokkenheid van patiënten bij medisch wetenschappelijk onderzoek. Tijdschr Gezondheidsr. 2016;94:92–102.

de Wit MP, Elberse JE, Broerse JE, Abma TA. Do not forget the professional - the value of the FIRST model for guiding the structural involvement of patients in rheumatology research. Health Expect. 2015b;18:489–503. doi:10.1111/hex.12048.

de Wit M, Kvien T, Gossec L. Patient participation as an integral part of patient reported outcomes development guarantees the representativeness of the patient voice – a case-study from the field of rheumatology. RMD Open. 2015a;1:e000129. doi:10.1136/rmdopen-2015-000129.

Dudley L, Gamble C, Allam A, Bell P, Buck D, Goodare H, et al. A little more conversation please? Qualitative study of researchers' and patients' interview accounts of training for patient and public involvement in clinical trials. Trials. 2015;16:190. doi:10.1186/s13063-015-0667-4.

Elberse JE, Caron-Flinterman JF, Broerse JE. Patient-expert partnerships in research: how to stimulate inclusion of patient perspectives. Health Expect. 2010;14(3):225–39. doi:10.1111/j.1369-7625.2010.00647.x.

Gagnon MP, Lepage-Savary D, Gagnon J, St-Pierre M, Simard C, Rhainds M, et al. Introducing patient perspective in health technology assessment at the local level. BMC Health Serv Res. 2009;9:54. doi:10.1186/1472-6963-9-54.

Gossec L, de Wit MPT, Heiberg T, Maccarone M, Balanescu A, Balint P et al. Elaboration and preliminary validation of the psoriatic arthritis impact of disease (psaid) questionnaire. A 13-country eular initiative with involvement of patient research partners from each country. Paper presented at the EULAR congress, Berlin, 2013.

Haywood K, Brett J, Salek S, Marlett N, Penman C, Shklarov S, et al. Patient and public engagement in health-related quality of life and patient-reported outcomes research: what is important and why should we care? Findings from the first ISOQOL patient engagement symposium. Qual Life Res. 2014;24(5):1069–76. doi:10.1007/s11136-014-0796-3.

Hewlett S, De Wit M, Richards P, Quest E, Hughes R, Heiberg T, et al. Patients and professionals as research partners: challenges, practicalities, and benefits. Arthritis Rheum. 2006;55:676–80. doi:10.1002/art.22091.

ICMJE Recommendations for the conduct, reporting, editing, and publication of scholarly work in medical journals. 2015. http://www.icmje.org/icmje-recommendations.pdf. Accessed 12 July 2015.

INVOLVE. Website supporting public involvement in NHS, public health and social care research. 2016. http://www.invo.org.uk/. Accessed 10 Feb 2016.

ISPOR Strategy for patient-oriented research. Canadian Institutes of Health Research. 2016. http://www.cihr-irsc.gc.ca/e/41204.html. Accessed 18 Oct 2016.

Kirwan J, de Wit M, Frank L, Haywood K, Salek S, Brace-McDonnell S et al. Patient as partners in health outcomes research: learnings from the field. Value Health. 2016;20(3):481–86.

Legare F, Boivin A, van der Weijden T, Pakenham C, Burgers J, Legare J, et al. Patient and public involvement in clinical practice guidelines: a knowledge synthesis of existing programs. Med Decis Mak. 2011;31:E45–74. doi:10.1177/0272989X11424401.

Mayer M. Seeking what matters: patients as research partners. Patient. 2012;5:71–4. doi:10.2165/11632370-000000000-00000.

Nierse CJ, Schipper K, van Zadelhoff E, van de Griendt J, Abma TA. Collaboration and co-ownership in research: dynamics and dialogues between patient research partners and professional researchers in a research team. Health Expect. 2012;15:242–54. doi:10.1111/j.1369-7625.2011.00661.x.

Pittens CA, Vonk Noordegraaf A, van Veen SC, Anema JR, Huirne JA, Broerse JE. The involvement of gynaecological patients in the development of a clinical guideline for resumption of (work) activities in the Netherlands. Health Expect. 2013; doi:10.1111/hex.12121.

Seale B. Patients as partners. Building collaborative relationships among professionals, patients, carers and communities. London: The King's Fund; 2016.

Shippee ND, Domecq Garces JP, Prutsky Lopez GJ, Wang Z, Elraiyah TA, Nabhan M, et al. Patient and service user engagement in research: a systematic review and synthesized framework. Health Expect. 2015;18:1151–66. doi:10.1111/hex.12090.

Staniszewska S, Brett J, Mockford C, Barber R. The GRIPP checklist: strengthening the quality of patient and public involvement reporting in research. Int J Technol Assess Health Care. 2011;27:391–9. doi:10.1017/S0266462311000481.

Staniszewska S, Haywood KL, Brett J, Tutton L. Patient and public involvement in patient-reported outcome measures: evolution not revolution. Patient. 2012;5:79–87. doi:10.2165/11597150-000000000-00000.

Teunissen GJ, Visse MA, Laan D, de Boer WI, Rutgers M, Abma TA. Patient involvement in lung foundation research: a seven year longitudinal case study. Health. 2013;5:320–30. doi:10.4236/health.2013.52A043.

van de Bovenkamp HM. The limits of patient power. Examining active citizenship in Dutch health care [dissertation]. Rotterdam: Erasmus University; 2010.

Wilson P, Mathie E, Keenan J, McNeilly E, Goodman C, Howe A, et al. ReseArch with patient and public invOlvement: a RealisT evaluation – the RAPPORT study. Health Serv Deliv Res. 2015; doi:10.3310/hsdr03380.

Chapter 9
Developing Patient-Reported and Relevant Outcome Measures

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9.1 PROM Development

This chapter will examine good practice guidance for patient-centred approaches towards PROM development. During the last decade, we have witnessed a paradigm shift in how outcomes are measured from a more clinical, physician-oriented perspective to a more patient-focused perspective, which has led to the emergence of the notion of patient-reported outcome (PRO). The concept of PRO seeks to understand how patients feel, function and live their lives in relation to health challenges and associated healthcare and is more encompassing than earlier terms, such as patient global assessment, health status, quality of life or symptom checklists. In this chapter, we argue that well-developed questionnaires, or PRO measures (PROMs), which reflect patients' perspectives, have the potential to provide valuable patient-based evidence in HTA. PROM development should engage with patients as participants (US Food and Drug 2009) and increasingly as research partners (Staniszewska et al. 2012; de Wit et al. 2013; Chap. 8) through all stages of development. This promotes patients as the determinants of the key constructs

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underpinning the PROM. This approach will support a transparent and auditable approach towards capturing patients' contributions to the measurement of relevant outcomes, thereby enhancing the face and content validity, relevance and acceptability of measures. In this chapter, we describe eight key stages in PROM development (Fig. 9.1) and reflect on how patients can participate in this process.

9.2 Key Stages in Developing a PROM

9.2.1 Establishing the Need for a New Measure

Developing a new PROM is a costly and time-consuming activity. Initially in relation to a HTA, efforts should be made to select a measure already available for the intended purpose, embarking upon development of a new measure only when there is an unmet need.

Systematic reviews of PROMs' availability, quality and acceptability are essential in supporting any decision to develop a PROM (Haywood et al. 2014a). If PROMs are available, one needs to establish if they are 'good' enough for the intended purpose, taking into consideration evidence of their development, relevance and acceptability as outlined above, alongside evidence of quality (Haywood et al. 2012; Terwee et al. 2007; Streiner et al. 2014) and consideration of their appropriateness for the proposed application.

9.2.2 Identifying Key Collaborators

From the beginning a new PROM should be developed with both the end users and intended application in mind. Key considerations comprise by whom, when and how the measure will be completed and who will receive the scores or analyses (such as HTA bodies). A team of experts is required throughout the development process including patient representatives, clinicians, clinical academics and measurement experts. However, if the new PROM is intended for use also by device manufacturers, health service or health technology developers and HTA bodies as the end users who will receive the scores for strategic and reimbursement decision-making, their representative should join the team of experts as additional stakeholders.

9.2.2.1 Core Research Team and Advisory Group

A small core research team, responsible for conducting the day-to-day research activities, should seek to include measurement experts, clinical academics, clinicians and patient research partners. A larger advisory group will include representatives

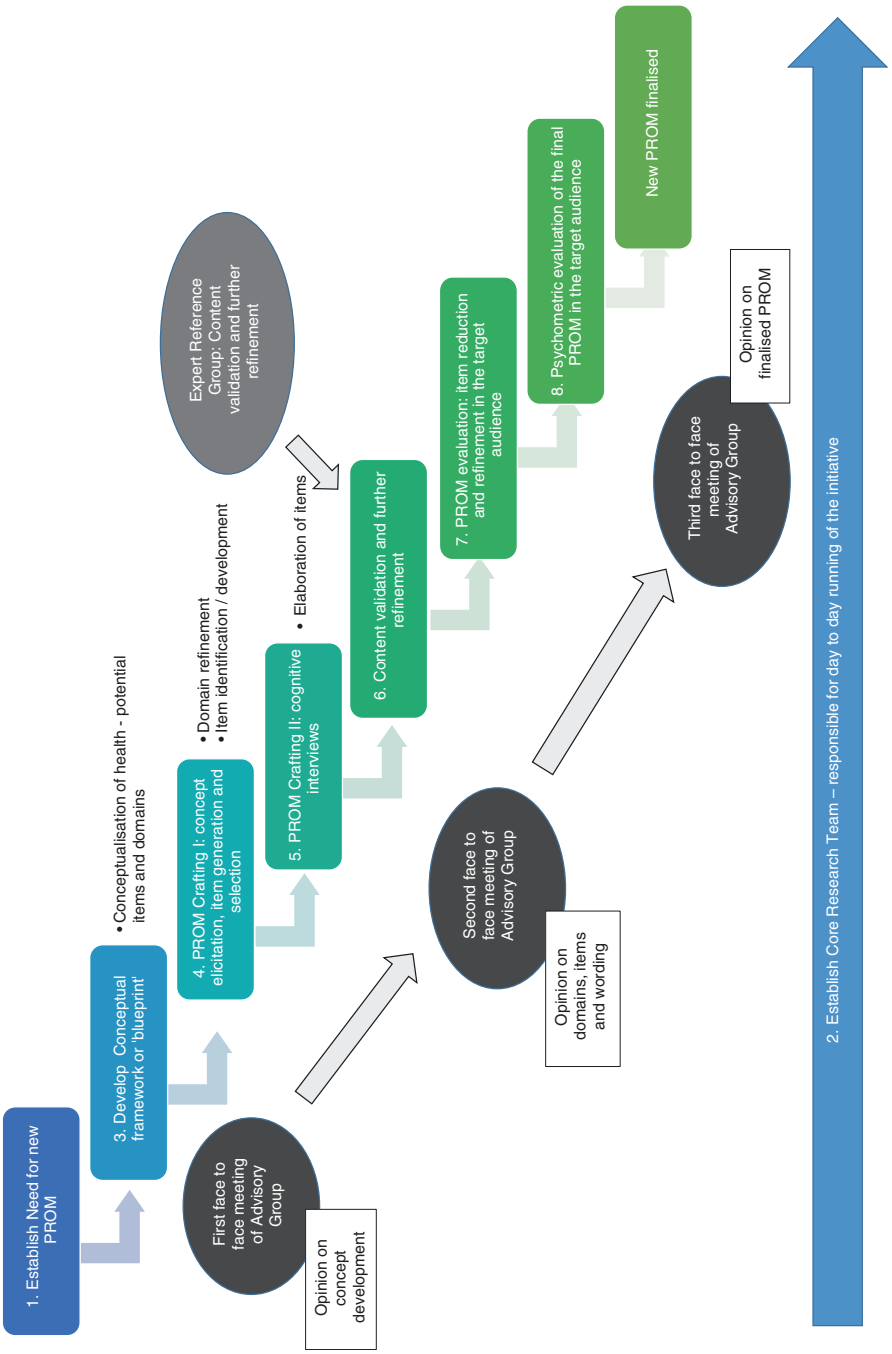

Fig. 9.1 Key stages in PROM development

from these same groups, with the addition of patient representatives, scientific organisation representatives, sponsors of the research and relevant health technology developer participants. In contrast to the core research team, the advisory group provides a more strategic oversight to the development of the PROM, commenting and contributing on each stage of the PROM development process.

9.2.2.2 Expert Reference Groups

Two external reference groups may also be established. These include (1) an expert patient reference group and (2) a professional expert group, both of whom will be utilising the measures and the information arising in their decision-making. These panels will be called upon at key stages in PROM development to comment on content, structure, format and appropriateness. An example of where these panels can play a critical role is in helping to find a resolution for the tension that may occur between the findings of the qualitative research (*Stage 9.2.4*) and the demands of the psychometric evaluation (*Stage 9.2.8*) (Gossec et al. 2014).

9.2.3 Developing a Conceptual Framework

Defining what a PROM is intended to measure is a crucial but often overlooked and poorly reported step in PROM development. Guidance has highlighted the importance of providing a clear conceptual framework of 'what' the PROM is intended to measure (US Food and Drug 2009; Patrick et al. 2011a).

A first step is to understand the medical, or disease, model of an illness (US Food and Drug 2009; Patrick et al. 2011a; Victorson et al. 2014), for example, the biology of the disease, associated symptoms and extent of impairment. This should underpin an appreciation of any potential patient-reported symptom and associated illness impact and hence the variables that may contribute to a developing biopsychosocial model of illness.

The conceptual framework describes the overriding concept of health underpinned by 'hypothesized relationships among items, domains and concepts measured' (US Food and Drug 2009, p. 9). That is, the specific questions (items) or groups of questions (domains) that should be considered for inclusion within a PROM to reflect the aspects of health (concepts) to be assessed. In effect, the conceptual framework is an 'organising tool that summarises what has been found in the literature and discussions with experts' (Patrick et al. 2011a, p. 971). It informs the developing topic guide for the qualitative research. Furthermore, it evolves as a consequence of findings from the qualitative research providing a 'blueprint' of the outcomes that really matter to patients with the target illness and hence the outcomes that should be considered for inclusion in the developing PROM (Parslow et al. 2015; Gorecki et al. 2010) (Fig. 9.2).

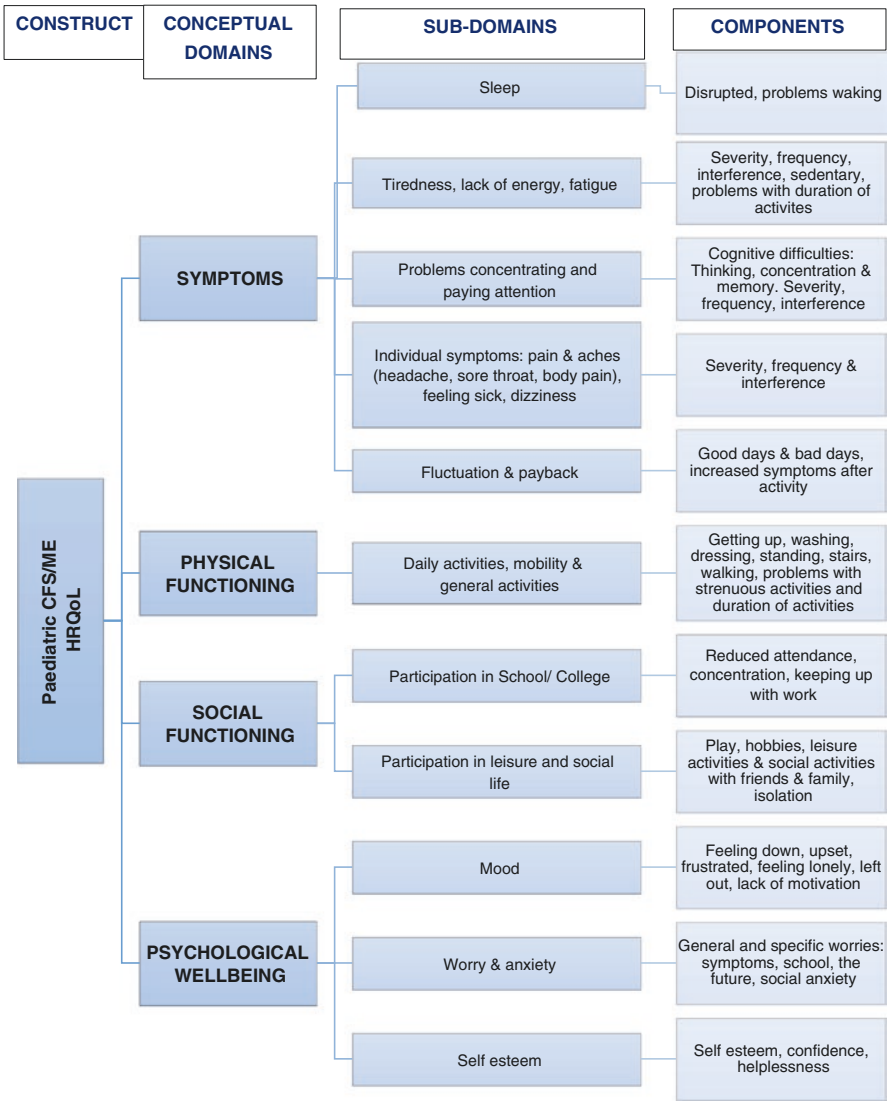

Fig. 9.2 A conceptual framework to underpin a new PROM for assessing the health-related quality of life (HRQoL) of children with chronic fatigue syndrome/myalgic encephalitis (CFS/ME) (Parslow 2016)

9.2.4 Crafting the PROM-I: Concept Elicitation, Item Generation and Selection

Current guidance on PROM development stipulates the importance of transparency in the data generation and analytical processes—creating a clear audit trail from concept elicitation to final items (US Food and Drug 2009; Patrick et al. 2011a).

9.2.4.1 Existing Measures

The content and focus of existing PROMs may contribute to both the developing conceptual framework and the list of potential items. For most instances of new PROM development, existing scales within the same disease or with a similar focus are available and should be reviewed.

Organisations such as the Patient-Reported Outcomes Measurement Information System (PROMIS) have established topic-specific 'item banks' (Health Measures 2016)—large numbers of items, or questions, derived from established measures and qualitative research with patients, but whose association has been determined by item response theory and hence form the basis of computer adaptive testing (CAT) approaches to PROM administration (Reeve et al. 2007). Such item banks can play a useful contribution to the development of new measures. For example, development of the Headache Impact Test (HIT) group of measures was informed by an item bank founded on several established migraine/headache measures and clinical judgement (Bjorner et al. 2003). Initial testing revised and reformatted the items and response formats to produce the CAT-HIT which has access to 54 items within the HIT item bank; a short-form, standardised version includes just six items—the HIT-6 (Kosinski et al. 2003).

9.2.4.2 Existing Literature

Systematic reviews and meta-syntheses of the qualitative literature can further assist in understanding the lived experience of patients and identifying relevant outcomes, contributing to the developing conceptual framework and item pool (e.g. Parslow et al. 2016).

9.2.4.3 Experts: Defining the Sample

The extent to which participants are representative of the target population and condition—considering variations in gender, age, disease severity and presentation—is essential to concept elicitation and item generation, ensuring content relevance and validity. For example, development of the EASi-QoL for Ankylosing Spondylitis (AS) included qualitative data generated from in-depth interviews with 29 patients and a UK survey of 462 patients (Haywood et al. 2010). Respondents identified the most important areas of their life affected by AS, ensuring that priorities and values representative of the wide spectrum of AS presentation and a broad socio-demographic mix contributed to concept elicitation and item generation.

Driven by changing global regulatory systems and HTA, it is increasingly recognised that for PROM data to have greater universality and relevance to a wide range of cultures, patients from different cultures and settings should be involved in item generation and selection. The result of such participation seeks to avoid culture-specific words or phrases and concepts that would be difficult to reproduce

cross-culturally. Models of PROM development which build in universality and translatability from the start are increasingly observed. For example, development of the PsAID questionnaire included 12 patient research partners from 12 European countries who were active through all stages of PROM development; all were fluent in English and had personal experience of psoriatic arthritis (Gossec et al. 2014). Moreover, the domain selection and external validation of the developing measure were further supported by an international cross-sectional study of 140 patients from ten countries who were invited to rank the domains in order of importance. Whilst it may not always be possible to achieve such integration, developers should be cognisant of the importance of these issues.

Increasingly social media is utilised to contribute to item generation and further development of PROMs. For example, an online forum of members of the hyperhidrosis patient organisation contributed to the generation of items for the Hyperhidrosis Quality of Life questionnaire (HidroQOL) (Kamudoni et al. 2015). Added benefits of this approach include the large number of international contributors, adding to the universality of the approach.

9.2.4.4 Qualitative Research

Rigorous qualitative research which seeks to better understand patients' perspectives and experiences is essential for concept elicitation and item generation so that PROMs are comprehensive and relevant to the target population (Brédart et al. 2014). A range of qualitative methods including semi-structured interviews, focus group discussions and modified Delphi surveys (Haywood et al. 2010; Gossec et al. 2014, Bartlett et al. 2012) can be used. However, this information is often poorly reported by developers (Patrick et al. 2011a). Recent guidance has highlighted the importance of transparency in both the qualitative approach and methods of data collection (US Food and Drug 2009; Patrick et al. 2011a). Where, historically, such qualitative exploration and analysis have been undertaken by academics or clinical researchers, patients are increasingly involved in this process as patient research partners (Gossec et al. 2014; Chap. 8).

9.2.4.5 Analysis of Qualitative Data: Quality Assurance in PROM Development

Data analysis seeks to refine the large amount of qualitative data into a long list of items that reflects the evolving conceptual framework in a manner that is transparent and meaningful and which ultimately supports the allocation of scores to enable quantification of the target construct. The data analysis should be both inductive—discovering new patterns and themes—and deductive, that is, regarding the evolving conceptual framework (Patrick et al. 2011a).

The analysis consists of several steps. First, the accuracy of the transcribed audio recordings should be checked to ensure preservation of the integrity of the generated

data (Patrick et al. 2011a; Golics et al. 2014). Data analysis seeks to use words and phrases generated by participants to craft the evolving concepts, themes and subthemes of the conceptual framework. Several trained researchers, or coders, should be involved in this process—working independently in the first instance, before discussing the developing themes to identify areas of consistency, inconsistency and concept saturation, a process which is repeated throughout data analysis. The transparent illustration of developing themes and codes, for example, on a thematic map, may assist with communicating data pattern conceptualisation. The thematic prevalence of a concept, that is, the number of patients expressing a concept, can also assist with item selection. For example, potential items were selected for the Family Reported Outcome Measure (FROM-16), a population-derived measure of the impact of illness on the partner or family members of patients, if mentioned by more than 5% of interviewees (Golics et al. 2014). Recent examples of PROM development have highlighted where patient partners, trained in qualitative data analysis, have actively collaborated with experienced coders in this process (Chap. 8).

Guidance suggests that the process of documenting concept saturation should be specified within the study protocol (US Food and Drug 2009; Patrick et al. 2011a). To demonstrate that concept saturation has been achieved, first attention must be paid to the representativeness of the population. Once this has been satisfactorily achieved, good practice supports the continuation of interviews with some additional 10–20% of patients before confirming saturation (Golics et al. 2014; Salek et al. 2016).

The use of computer-assisted qualitative data analysis software programmes, for example, NVivo, facilitates the data management, the assessment of between-coder reliability and the documentation of concept saturation and aids quality assurance audits (Patrick et al. 2011a). Data analysis creates a model for the data that makes the data understandable by the research team in the next stage.

9.2.4.6 Item Crafting: Generation and Selection

Once the analysis is complete, the core research team seeks to further refine the conceptual framework, developing domains and subdomains from the defined themes and subthemes and crafting specific questions, or ‘items’, with which to populate an initial long-form version of the developing PROM. Item crafting seeks to convert long, transcribed text into comprehensible, jargon-free, easy-to-read, specific and universal statements which link the essence of the patients’ experience with the content of the developing PROM. The target concept and purpose of measurement must be closely adhered to during this process (Patrick et al. 2011b); clearly specified item selection criteria can assist in guiding the appropriateness of developing items.

The large amounts of data generated at this stage often results in too many potential themes and associated items. The process of item selection is an iterative one, during which multiple viewpoints should be considered and integrated—including the qualitative data, the multidisciplinary team, patient research partners and meth-

odological experts. An important challenge is to avoid losing the patient perspective, and strategies to ensure that the patient voice is retained should be considered. For example, involving patients in the prioritisation of the most important themes can assist in the process of refining the conceptual model and shortlisting items (Gossec et al. 2014).

9.2.4.6.1 Recall Period

The appropriateness of the recall period, that is, the timeframe against which a specified concept is considered, requires special attention. A range of variables including the target population, objectives and frequency of assessment and the content and frequency of an event may influence the appropriateness of the recall period. Commonly used recall periods include 'current time' and shorter periods such as the 'past week'. For example, if the PROM is used in research scenarios such as clinical trials, a recall period which captures an individual's experience 'at the present time' could be more appropriate.

9.2.4.6.2 Response Options and Scaling

The ability to communicate the subjective, qualitative experiences of the patient as an objective, numerical value is a central tenet of PROM development. Selection of an appropriate numerical scale with which to capture the patient experience is a crucial step. A large number of response scales are available, including categorical and adjectival, Likert-type, numerical rating and visual analogue scales (Streiner et al. 2014; Patrick et al. 2011b).

The appropriate number of response options in a scale is driven by a balance between accuracy and practicality. The greater the number of options will enhance the ability of the patient to communicate their experience, thus enhancing precision and discriminant validity, whilst also increasing reliability and responsiveness (Streiner et al. 2014). However, a smaller number of response options improve practicality: good practice supports the adoption of between five and seven responses (Streiner et al. 2014). The interval between each response option needs to be logical and 'equal' so that there is a gradual progression from one end of the scale to the other. Whilst there are other schools of thought that challenge this approach (e.g. Andrich 2011), this continues to be a common practice as an initial attempt for scaling of a newly developed PROM.

In arriving at the final score, for most PROMs, a simple summation of item scores is often described. Dependent on the context in which the PROM will be used. For example, at an individual or aggregate level, the final score can be represented either as the actual score or as a percentage. For PROMs which may be utilised within a routine practice setting, a further driver when considering the appropriateness and acceptability of response scales is the ability to score the final PROM and provide timely, interpretable and meaningful data to both clinicians and patients.

9.2.4.6.3 Mode of Administration

Patient self-completion is the preferred format for PROM administration and is a crucial consideration at the start of PROM development. However, there are instances—such as for patients with cognitive impairment or for young children—where proxy completion, such as by a caregiver, is essential (Haywood et al. 2014b).

9.2.4.6.4 Engaging with Experts

PROM development is an iterative process which requires several stages of drafting, evaluation and further refinement (Patrick et al. 2011b). The potential suitability of developing items and item stems, suitability of phraseology, recall period(s) and response scales should be explored with members of the advisory group. Insight from patients, experienced clinicians and measurement experts will help to refine the items—seeking to group, merge, order or delete items and endorse or refine domain development. This process will result in a long-form PROM suitable for cognitive interviewing.

9.2.5 Crafting the PROM-II: Cognitive Interviews

This stage represents the last opportunity for significant revision to the PROM (Patrick et al. 2011b). The focus of the cognitive interviews is to verify the relevance, acceptability, comprehension and comprehensiveness of the new PROM with participants' representative of the target population (Brédart et al. 2014; Patrick et al. 2011b; Hay et al. 2014). Four stages of cognitive processing should underpin the interviewing process: comprehension, the process of making sense of the question and developing a response; memory retrieval, the process of relevant information to enable a response; judgement, the process to determine if memory retrieval is accurate and complete; and response mapping, the process by which an appropriate response option is selected (Tourangeau 1984; Patrick et al. 2011b; Gorecki et al. 2012; Hay et al. 2014).

The two most commonly used interview techniques include 'thinking aloud', where respondents express aloud their thought processes whilst answering the question, and often followed by 'verbal probing', where respondents are invited to retrospectively paraphrase or rephrase items (Christodoulou et al. 2008; Brédart et al. 2014). Most authors describe several rounds of semi-structured interviews during which the patient completes either a subset of items or the full PROM (Haywood et al. 2010; Gorecki et al. 2010; Hay et al. 2014)—with both the patient and interviewer highlighting items or aspects of completion which are judged to be difficult or confusing, warranting further exploration. During this process, interviewers should pay careful attention to both verbal and non-verbal respondent clues. Whilst there is no standard approach for using cognitive interview data

for PROM modification (Christodoulou et al. 2008; Gorecki et al. 2012), good practice supports the exploration of results from each round with 'experts' (Haywood et al. 2010; Gorecki et al. 2012; Hay et al. 2014), for example, the core research team or advisory group. Where significant revisions are made, subsequent interview rounds will be required. A summary report of the interviewing process should highlight changes made to the PROM. The number of interviewees per round varies, with total sample size estimates ranging from 7 (Leidy and Vernon 2008) to more than 100 with three rounds of interviewing (Hay et al. 2014). The goal is to achieve consensus from a group of patients that the PROM is appropriate.

The ability of patients with different literacy levels to accurately and adequately complete the PROM is a key consideration at this stage of development (Streiner et al. 2014; Petkovic et al. 2015). Sophisticated software—using readability formulas such as Flesch reading ease, FOG and FORECAST—is available with which to evaluate PROM readability (e.g. Zraick and Atcherson 2012), providing a useful adjunct to the cognitive interviewing process.

9.2.6 Content Validation and Further Refinement

Further exploration of the content validity of the developing measure seeks to ascertain that the focus and emphasis of the measure is fit for purpose (Patrick et al. 2011b; Rothman et al. 2009). Developers have adopted different approaches in seeking to establish PROM content validity. For example, the developers of the HidroQoL (Kamudoni et al. 2014) and FROM-16 (Golics et al. 2014) utilised modified nominal groups. First, copies of the developing PROM and a content validation questionnaire were sent to two expert panels—one formed of patients and the second of clinicians. Participants were asked to rate the PROM for language clarity, completeness, relevance and appropriateness of response scale using a 4-point Likert scale for agreement. These groups then met separately to discuss the results and reach consensus on proposed refinements. Agreement between panel members was reported both quantitatively and qualitatively, supporting the process of content validation and informing PROM refinement.

This process results in the final long-form version of the PROM which will be evaluated in the target population.

9.2.7 PROM Evaluation: Item Reduction and Refinement in the Target Population

Item reduction is an important next step in refining the long-form PROM (Streiner et al. 2014). A preliminary psychometric evaluation should be undertaken using both traditional psychometrics (classical test theory) (US Food and Drug 2009;

Streiner et al. 2014) and modern psychometric methods such as Rasch measurement theory (Hobart and Cano 2009) or item response theory (Streiner et al. 2014; Reeve et al. 2007). The importance of this step is to realise a set of items contributing to the measurement of the concept of interest and to elucidate on the internal structure of the new measure.

9.2.7.1 Sample and Sample Size

The initial evaluation should be undertaken in a large, representative population of patients with the target condition. Purposive sampling should be undertaken to ensure that patients representative of key disease features, severity levels and socio-demographic variables are included.

Sample size guidance for 'new' summated scales suggests a minimum of five to ten subjects per item (Blazeby et al. 2002). For example, for a new measure with several potential domains, the longest of which includes ten potential items, 100 patients will be required. The subject to item ratio is a frequently used method to determine a required sample size to perform exploratory and confirmatory factor analysis (E/CFA). However, guidance for sample size calculations for performing EFA ranges from 2 to 20 subjects per item, depending on the nature of the data (i.e. the stronger the data, the smaller the required sample size). Recent guidance from COSMIN[1] supports a more conservative maximum number of seven subjects per item or an absolute minimum total of 100 subjects (Terwee et al. 2012). Modern psychometrics requires consideration of the impact of sample size on item fit statistics which, when using polytomous data, are highly sensitive to sample sizes (Streiner et al. 2014). In general, as large a sample size as possible is ideal (Streiner et al. 2014), with a sample size of up to 250 recommended to produce a statistically stable measure.

9.2.7.2 Analyses: Traditional and Modern

Traditional analyses should seek to establish preliminary evidence in support of the acceptability, data quality (scaling assumptions) and internal structure of the measure. Modern psychometrics contribute to this understanding, with the addition of a further exploration of scale targeting, item response, item fit and response bias to further guide PROM refinement and identification of items with poor psychometric properties which are considered for removal. These analyses and comparisons between both approaches are further elucidated by Gorecki et al. (2013) (Table 1, p 4–5). This will result in the final version of the PROM for which final, further psychometric evaluation in the target population is required.

[1] Consensus-based standards for the selection of health measurement instruments

9.2.8 Psychometric Evaluation of the Final PROM in the Target Population

Finally, a comprehensive psychometric evaluation of the final version PROM is required in a large, independent and representative population to confirm evidence of quality, relevance and acceptability. The precision of the PROM depends on the quality of the psychometric evaluation and the evidence of measurement properties. Psychometric evaluations should include the following.

9.2.8.1 Reliability (Internal Consistency; Test-Retest; Measurement Error)

Evaluation of reliability considers the degree of measurement error and is central to the measurement process (Streiner et al. 2014). For example, poor reliability may obscure the correlation of a measure with other measures in the assessment of convergent validity. Similarly, a measure's ability to detect change over time, responsiveness, is equally effected by poor measurement reliability. For multi-item PROMs, both the internal consistency (inter-item correlations, item-partial total correlations and Cronbach's alpha coefficient) and test-retest reliability should be evaluated. Measurement reliability is affected by the target population and setting in which it is completed and hence should be re-established each time a measure is put to new use.

9.2.8.2 Validity (Internal Analyses and Analyses Against External Criteria)

Evaluation of measurement validity seeks to establish evidence in support of the proposed measurement construct. Although delineation is made between different types of validity (content, criterion and construct), a unified perspective considers all forms of validity to be encompassed by construct validity (Streiner et al. 2014). Construct validity relates to the extent to which theoretically derived hypotheses relating to the construct being measured by a PROM are supported by empirical evidence. As there is no single 'ultimate test' for construct validity (Streiner et al. 2014), its assessment involves testing for various hypotheses relating to the relationship between the underlying variable and the items of the PROM in different situations. Therefore, assessing PROM validity requires the testing of a number of clearly specified hypotheses (Terwee et al. 2012; Mokkink et al. 2010).

9.2.8.3 Responsiveness (Criterion or Construct-Based Assessment)

The assessment of responsiveness, also referred to as longitudinal validity, requires an external measure as a criterion for determining whether the patient's condition has changed, improved or deteriorated (Streiner et al. 2014). Establishing evidence

of PROM responsiveness requires not only showing that a PROM can capture statistically significant changes (changes beyond chance) but more importantly that it can capture minimal changes considered important by the patient (Mokkink et al. 2010). The hypotheses to be considered when testing the new PROM include:

1. If the new PROM can capture change in the group of patients experiencing minimal but important change in the condition.
2. If the magnitude of change in patients with minimal improvement in their condition is greater than those with no change in their condition.
3. If change will be greater over the longer period in those patients receiving active treatment.

9.2.8.4 Interpretability

The qualitative meaning of PROM scores is not intuitively apparent (de Vet et al. 2006); the credibility and usefulness of such data are dependent on interpretative guidance and its appropriate use. The cross-sectional comparison of between group 'differences'—also referred to as 'minimal important difference' (MID)—in scores for clearly defined groups can facilitate score interpretation, for example, comparing score differences between the general population and patients with inflammatory rheumatic disease (Salaffi et al. 2009) or between groups categorised according to mild, moderate or severe levels of impact of a condition (Hongbo et al. 2005).

However, interpretation of change scores is crucial to understanding if an individual's health has improved or deteriorated to an extent that warrants a change in treatment. Two values are important in this context (de Vet et al. 2006): (1) the smallest detectable change (SDC), a change that is greater than measurement error, and (2) the minimal important change (MIC), 'the smallest difference in score … which patients perceive as beneficial' (Jaeschke et al. 1989, p. 408). Consensus is lacking on the most appropriate evaluation of MIC, but both anchor-based—which adopts an external anchor which specifies 'minimal important'—and distribution-based approaches are described (Crosby et al. 2003). Recent guidance emphasises the importance of understanding meaningful change at the individual level (i.e. the responder), recommending estimation of a 'responder definition' based on an empirically derived minimally important change (MIC) (US Food and Drug 2009).

In addition, evidence which supports MID and MIC interpretation adds to the robustness of the measure and its utility both at individual and aggregate level. For example, HTA appraisal of PROM data for a new product compared with 'standard of care,' where MIDs are used to demonstrate a between group difference which is important to patients, would be important evidence to facilitate reimbursement recommendations in favour or against the product.

9.2.8.5 Acceptability and Feasibility

Evidence for practical properties including acceptability (relevance and respondent burden) and practicality (completion time, cost, etc.) should also be documented.

9.3 Concluding Remarks

Well-developed PROMs seek to ensure that research and decision-making better capture patient-derived evidence about how they feel, function and live their lives, often aiming to provide a standardised, relevant and acceptable assessment of this experience. Good practice guidance recommends the use of both generic and disease-specific measures in HTA evaluations. However, for many patients, generic measures such as the EuroQoL EQ-5D may lack relevance (Haywood et al. 2016). In recent years, approaches that support the 'mapping' of scores from disease-specific PROMs into utility values for the purpose of economic appraisal and HTA evaluations have been developed (Longworth and Rowen 2013). This has the advantage of moving away from utilising a generic measure alongside a disease-specific measure, as has been a common practice. However, HTA appraisal should use PROMs to assist in their decision-making for reimbursement and not just to inform economic appraisal. Although the quality and quantity of life is built in to cost-effectiveness analyses, it does not entirely reflect the impact of the health technology on what patients can and cannot do. HTA should be more cognisant of the value of PROMs in their own right—that is, in isolation from their use in economic appraisal. The selection of well-developed, patient-derived PROMs, developed in a way that reflects the key stages discussed in this chapter, will support this and can provide high-quality, robust patient-based evidence to contribute to HTA.

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References

Andrich D. Rating scales and Rasch measurement. Expert Rev Pharmacoecon Outcomes Res. 2011;11:571–85.

Bartlett SJ, Hewlett S, Bingham III CO, Woodworth TG, Alten R, Pohl C, OMERACT RA Flare Working Group, et al. Identifying core domains to assess flare in rheumatoid arthritis: an OMERACT international patient and provider combined Delphi consensus. Ann Rheum Dis. 2012;71:1855–60.

Brédart A, Marrel A, Abetz-Webb L, Lasch K, Acquadro C. Interviewing to develop patient-reported outcome (PRO) measures for clinical research: eliciting patients' experience. Health Qual Life Outcomes. 2014;5:12–5.

Bjorner JB, Kosinski M, Ware Jr JE. Calibration of an item pool for assessing the burden of headaches: an application of item response theory to the headache impact test (HIT). Qual Life Res. 2003;12:913–33.

Blazeby J, Sprangers MA, Cull A, Groenvold M, Bottomley A. EORTC quality of life group: guidelines for developing questionnaire modules. 3rd ed. Brussels: EORTC Quality of Life Group Publication ; 2002.2-930064-24-2

Crosby RD, Kolotkin RL, Williams GR. Defining clinically meaningful change in health-related quality of life. J Clin Epidemiol. 2003;56:395–407.

Christodoulou C, Junghaenel DU, DeWalt DA, Rothrock N, Stone AA. Cognitive interviewing in the evaluation of fatigue items: results from the patient-reported outcomes measurement information system (PROMIS). Qual Life Res. 2008;17:1239–46.

de Vet HC, Terwee CB, Ostelo RW, Beckerman H, Knol DL, Bouter LM. Minimal changes in health status questionnaires: distinction between minimally detectable change and minimally important change. Health Qual Life Outcomes. 2006;22:54.

de Wit M, Abma T, Koelewijn-van Loon M, Collins S, Kirwan J. Involving patient research partners has a significant impact on outcomes research: a responsive evaluation of the international OMERACT conferences. BMJ Open. 2013;3(5):e002241.

Golics CJ, Basra MK, Finlay AY, Salek S. The development and validation of the Family Reported Outcome Measure (FROM-16)© to assess the impact of disease on the partner or family member. Qual Life Res. 2014;23:317–26.

Gorecki C, Lamping DL, Brown JM, Madill A, Firth J, Nixon J. Development of a conceptual framework of health-related quality of life in pressure ulcers: a patient-focused approach. Int J Nurs Stud. 2010;47:1525–34.

Gorecki C, Lamping DL, Nixon J, Brown JM, Cano S. Applying mixed methods to pretest the Pressure Ulcer Quality of Life (PU-QOL) instrument. Qual Life Res. 2012;21:441–51.

Gorecki C, Brown JM, Cano S, Lamping DL, Briggs M, Coleman S, et al. Development and validation of a new patient-reported outcome measure for patients with pressure ulcers: the PU-QOL instrument. Health Qual Life Outcomes. 2013;11:95.

Gossec L, de Wit M, Kiltz U, Braun J, Kalyoncu U, Scrivo R, EULAR PsAID Taskforce, et al. A patient-derived and patient-reported outcome measure for assessing psoriatic arthritis: elaboration and preliminary validation of the Psoriatic Arthritis Impact of Disease (PsAID) questionnaire, a 13-country EULAR initiative. Ann Rheum Dis. 2014;73:1012–9.

Hay JL, Atkinson TM, Reeve BB, Mitchell SA, Mendoza TR, Willis G, NCI PRO-CTCAE Study Group, et al. Cognitive interviewing of the US National Cancer Institute's patient-reported outcomes version of the common terminology criteria for adverse events (PRO-CTCAE). Qual Life Res. 2014;23:257–69.

Haywood KL, Garratt AM, Jordan K, Healey EL, Packham JC. Evaluation of ankylosing spondylitis quality of life (EASi-QoL): reliability and validity of a new patient-reported outcome measure. J Rheumatol. 2010;37:2100–9.

Haywood KL, Staniszewska S, Chapman S. Quality and acceptability of patient reported outcome measures in chronic fatigue syndrome/Myalgic encephalitis (CFS/ME): a structured review. Qual Life Res. 2012;21:35–52.

Haywood KL, Collins S, Crawley E. Assessing severity of illness and outcomes of treatment in children with Chronic Fatigue Syndrome/Myalgic Encephalitis (CFS/ME): a systematic review of patient-reported outcome measures. Child Care Health Dev. 2014a;40:806–24.

Haywood KL, Whitehead L, Perkins GD. The psychosocial outcomes of cardiac arrest: relevant and robust patient-centred assessment is essential. Resuscitation. 2014b;85:718–9. doi:10.1016/j.resuscitation.2014.03.305.

Haywood KL, Wilson R, Staniszewska S, Salek S. Using PROMs in healthcare: who should be in the driving seat–policy makers, health professionals, methodologists or patients? Patient. 2016;9(6):495–8.

Health Measures. Applications in reserach. 2016. http://www.nihpromis.org/researchers/researchershome. Accessed 21 Dec 2016.

Hobart J, Cano S. Improving the evaluation of therapeutic interventions in multiple sclerosis: the role of new psychometric methods. Health Technol Assess. 2009;13:1–177.

Hongbo Y, Thomas CL, Harrison MA, Salek MS, Finlay AY. Translating the science of quality of life into practice: what do dermatology life quality index scores mean? J Invest Dermatol. 2005;125:659–6.

Jaeschke R, Singer J, Guyatt G. Measurement of health status. Ascertaining the minimal clinically important difference. Control Clin Trials. 1989;10:407–15.

Kamudoni P, Mueller B, Salek MS. The development and validation of a disease-specific quality of life measure in hyperhidrosis: the hyperhidrosis quality of life index (HidroQOL©). Qual Life Res. 2015;24:1017–27.

Kosinski M, Bayliss MS, Bjorner JB, Ware Jr JE, Garber WH, Batenhorst A, et al. A six-item short-form survey for measuring headache impact: the HIT-6. Qual Life Res. 2003; 12:963–74.

Leidy N, Vernon M. Perspectives on patient-reported outcomes. Content validity and qualitative research in a changing clinical trial environment. PharmacoEconomics. 2008;26:363–70.

Longworth L, Rowen D. Mapping to obtain EQ-5D utility values for use in NICE health technology assessments. Value Health. 2013;16:202–10.

Mokkink LB, Terwee CB, Patrick DL, Alonso J, Stratford PW, Knol DL, et al. The COSMIN study reached international consensus on taxonomy, terminology, and definitions of measurement properties for health-related patient-reported outcomes. J Clin Epidemiol. 2010;63:737–45.

Parslow R, Patel A, Beasant L, Haywood KL, Johnson D, Crawley E. What matters to children with CFS/ME? A conceptual model as the first stage in developing a PROM. Arch Dis Child. 2015;100:1141–7. doi:10.1136/archdischild-2015-308831. Epub 2015 Oct 9

Parslow R, Harris S, Broughton J, Alattas A, Crawley E, Haywood K, et al. Children's experiences of Chronic Fatigue Syndrome/Myalgic Encephalomyelitis (CFS/ME): A systematic review and meta-ethnography of qualitative studies. BMJ Open. 2016;7(1):e012633.

Parslow RM. (2016). Developing a Patient Reported Outcome Measure (PROM) for Children with Chronic Fatigue Syndrome/Myalgic Encephalomyelitis (CFS/ME). Thesis submitted to the University of Bristol.

Patrick DL, Burke LB, Gwaltney CJ, Leidy NK, Martin ML, Molsen E, et al. Content validity-establishing and reporting the evidence in newly developed patient-reported outcomes (PRO) instruments for medical product evaluation: ISPOR PRO good research practices task force report: part 1-eliciting concepts for a new PRO instrument. Value Health. 2011a;14:967–77.

Patrick DL, Burke LB, Gwaltney CJ, Leidy NK, Martin ML, Molsen E, et al. Content validity-establishing and reporting the evidence in newly developed patient-reported outcomes (PRO) instruments for medical product evaluation: ISPOR PRO good research practices task force report: part 2-assessing respondent understanding. Value Health. 2011b;14:978–88.

Petkovic J, Epstein J, Buchbinder R, Welch V, Rader T, Lyddiatt A, et al. Toward ensuring health equity: readability and cultural equivalence of OMERACT patient-reported outcome measures. J Rheumatol. 2015;42:2448–59.

Reeve BB, Hays RD, Bjorner JB, Cook KF, Crane PK, Teresi JA, PROMIS Cooperative Group, et al. Psychometric evaluation and calibration of health-related quality of life item banks: plans for the patient-reported outcomes measurement information system (PROMIS). Med Care. 2007;45(5 Suppl 1):S22–31.

Rothman M, Burke L, Erickson P, Leidy NK, Patrick DL, Petrie CD. Use of existing patient-reported outcome (PRO) instruments and their modification: the ISPOR good research practices for evaluating and documenting content validity for the use of existing instruments and their modification PRO task force report. Value Health. 2009;12:1075–83.

Salaffi F, Carotti M, Gasparini S, Intorcia M, Grassi W. The health-related quality of life in rheumatoid arthritis, ankylosing spondylitis, and psoriatic arthritis: a comparison with a selected sample of healthy people. Health Qual Life Outcomes. 2009;7:25.

Salek S, Kamudoni P, Oliva E, Ionova T. Quality of life issues important to patients with haematological malignancies. Value Health. 2016;18:A709.

Staniszewska S, Haywood KL, Brett J, Tutton L. Patient and public involvement in patient-reported outcome measures: evolution not revolution. Patient. 2012;5:79–87.

Streiner DL, Norman GR, Cairney J. Health measurement scales: a practical guide to their development and use. 5th ed. UK: Oxford University Press; 2014.

Terwee CB, Bot SD, de Boer MR, van der Windt DA, Knol DL, Dekker J, et al. Quality criteria were proposed for measurement properties of health status questionnaires. J Clin Epidemiol. 2007;60:34–42.

Terwee CB, Mokkink LB, Knol DL, Ostelo RW, Bouter LM, de Vet HC. Rating the methodological quality in systematic reviews of studies on measurement properties: a scoring system for the COSMIN checklist. Qual Life Res. 2012;21:651–7.

Tourangeau R. Cognitive science and survey methods. In: Jabine T, Straf M, Tanur J, & Tourangeau R (Eds.). Cognitive aspects of survey methodology: Building a bridge between disciplines. Washington, DC: National Academy Press. 1984. pp. 73–100.

US Food and Drug. Administration guidance for industry: patient-reported outcome measures: use in medical product development to support labeling claims. Rockville: Department of Health and Human Services, Food and Drug Administration, Centre for Drug Evaluation and Research; 2009. http://www.fda.gov/downloads/Drugs/Guidances/UCM193282.pdf. Accessed 30 Jan 2016

Victorson DE, Cella D, Grund H, Judson MA. A conceptual model of health-related quality of life in sarcoidosis. Qual Life Res. 2014;23:89–101.

Zraick RI, Atcherson SR. Readability of patient-reported outcome questionnaires for use with persons with dysphonia. J Voice. 2012;26:635–41.

Chapter 10
Discrete Choice Experiments

Antje Tockhorn-Heidenreich, Mandy Ryan, and Rodolfo Hernández

10.1 Introduction

Discrete choice experiments (DCEs) were introduced into health economics in the early 1990s to ensure a person-centred approach to economic evaluation. While economic evaluation had focused on clinical outcomes, using measures such as quality-adjusted life years (QALYs), DCEs valued broader aspects of care, including how the care is delivered and non-health outcomes. DCEs are an attribute-based measure of value. They can be used to address a range of questions faced by decision-makers, including valuing patient experiences alongside clinical outcomes, valuing health states and predicting uptake/acceptability of new services. DCEs provide potentially useful information within a health technology assessment (HTA) decision-making context — what characteristics (attributes) are important, how important these are, trade-offs between characteristics, monetary measures of value and predictions of take-up rates and acceptability of interventions/medicines/services. This chapter provides an introduction to DCEs, summarising the method and discussing how they can inform the HTA process. The method is illustrated using a DCE within a HTA looking at surveillance of ocular hypertension. We discuss methodological challenges within a HTA context and areas for future research.

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10.2 Background

DCEs were introduced into health economics in the early 1990s to value aspects of healthcare beyond clinical outcomes (Ryan et al. 2008; de Bekker-Grob et al. 2012). Their application has increased to address a broad range of questions faced by decision-makers, including valuing patient experiences alongside clinical outcomes, valuing health states and predicting uptake/acceptability of new services. DCEs have also been shown to be informative within the HTA framework (e.g. McCormack et al. 2005; Robson et al. 2009; Burr et al. 2012; Adams et al. 2015; Morgan et al. 2015).

Interest in the use of DCEs has been increasing amongst decision-making bodies. For example, DCEs are mentioned as a potential method to elicit preferences in the guidelines of the Australian Pharmaceutical Benefits Advisory Committee (PBAC) (Australian Government, Department of Health 2014). The Institute for Quality and Efficiency in Health Care (IQWiG) in Germany explored the application of DCEs to inform relevant endpoints of a technology (IQWiG 2015) and initiated a pilot study to explore the application of DCEs to the assessment of new technologies (Mühlbacher et al. 2016). In England and Wales, the National Institute for Health and Care Excellence (NICE) accepts the need to move to monetary measures of value (which can be generated from DCEs) in the evaluation of public health interventions (NICE 2012). A European initiative involving industry, HTA bodies and the European Medicines Agency (EMA) explored how to establish a preference elicitation framework that captures patients' values in the appraisal of new technologies (IMI-2-PROTECT 2015). The US Food and Drug Administration (FDA) Patient Preference Initiative encourages medical device manufacturers to include information about trade-offs when evaluating the benefits and risks of treatment options, advocating DCEs with an example estimating 'minimum acceptable effectiveness' and 'maximum acceptable risk' for a weight-loss device (FDA 2016).

10.3 What Are DCEs?

DCEs assume that individuals' valuation of a health technology or medicine is based on its characteristics or attributes. For instance, in our case study below, the value of an ocular hypertension monitoring strategy is based on timely detection (to avoid progression of glaucoma), side effects of treatment, where monitoring takes place (hospital or local optician), and experience of the healthcare interaction (if the patient feels at ease and/or understands the purpose of monitoring). DCEs present survey respondents with a series of hypothetical choices, each described by the set of attributes that take different levels for each alternative. Figure 10.1 presents an example choice task. Respondents are presented with a number of such choices, determined by the experimental design (see below).

DCE Case Study: Preferences for Ocular Hypertension Monitoring

Background: Glaucoma is an eye condition caused by damage to the optic nerve, which can lead to profound sight loss or blindness. Ocular hypertension (OHT) is the only treatable risk factor — lowering intraocular pressure by medication, laser or surgery reduces the risk of glaucoma-related sight loss. Long-term monitoring of OHT provided by a specialist in secondary care or primary care was recommended by NICE (2009). Controversy resulted, with concern that hospital eye services would be overwhelmed by following up patients with low risk of sight loss. Little was known about how the public valued OHT monitoring.

Methods: Burr et al. (2012) conducted a DCE to investigate preferences for glaucoma monitoring. The DCE was part of a broader project concerned with optimal monitoring, funded by the UK National Institute of Health Research HTA programme. Figure 10.1 shows an example DCE choice.

Attributes *Alternatives*

	Monitoring Service A	Monitoring Service B	No monitoring Service
Number of people out of 10000 developing glaucoma in 10 years	740	1410	1600
Number of people out of 10000 developing severe glaucoma in 10 years	25	130	180
Number of people out of 10000 developing visual impairment in 10 years	2	15	25
Unwanted effects of treatment	None	Severe	None
Communication and understanding of information provided by the health professional	Made me feel at ease and made sure I understood the purpose of monitoring	Did not make me feel at ease and did not make sure I understood the purpose of monitoring	Not applicable
Place of testing	Hospital eye clinic	Local optician	No testing
Cost per year	£15/year	£30/year	No cost
	Service A	Service B	No Service
(tick one Box only)	☐	☐	☐

Levels

Fig. 10.1 Example choice set

Willingness to pay (WTP), a monetary measure of value, was generated for individual attributes of the DCE as well as different configurations of monitoring services. WTP values were incorporated into a decision model, and results from a cost-benefit analysis (CBA) were compared to a cost-utility analysis (CUA, cost per QALY).

Results and discussion: The CUA indicated that treating all OHT individuals with advice for an annual eye test to check intraocular pressure was the most likely cost-effective. However, the DCE highlighted the importance of having an active monitoring programme, with biennial monitoring at a hospital setting providing the highest incremental net benefit in the CBA. The CUA and CBA thus suggested different monitoring strategies.

When responding to DCEs, it is assumed that respondents consider all attributes and make trade-offs between them. These trade-offs express how much the respondent is willing to give up of one attribute (e.g. higher risk of glaucoma progression) to get more (e.g. better communication) or less (e.g. unwanted treatment effects) of another attribute. If a cost attribute is included, trade-offs can be expressed as WTP for marginal changes in attributes (see Table 10.1). Thus, valuation is measured in money as a common unit. This ensures that attributes can be compared in terms of their desirability. The derived WTP estimates can be used in a CBA framework to provide policy advice (McIntosh 2006). For example, WTP for an improved monitoring experience (e.g. feeling at ease and understand the monitoring process) can be compared to the costs of providing this service.

10.4 How Do DCEs Work?

Analysts follow four main stages to conduct a DCE (Lancsar and Louviere 2008, Bridges et al. 2011).

10.4.1 Stage 1: Identification of Attributes and Levels

Identification of the attributes to be compared may be based on literature reviews, validated quality of life instruments (e.g. the SF-6D utility instrument generated from the SF-6D profile measure), experts' opinions, interviews, focus groups or behavioural theory (e.g. economic theory predicts higher-income people are willing to pay more, reflected in the cost attribute). Attributes need to be (i) relevant to the target group, (ii) realistic in the considered context and (iii) tradable against each other. As a rule of thumb, all attributes that fulfil these criteria and impact treatment choices should be considered, because their inclusion in the DCE is likely to affect

analysts' understanding of elicited preferences (Lancsar and Louviere 2008). This is also important, because the exclusion of relevant attributes potentially biases estimates and might result in suboptimal policy advice. Quantitative and qualitative pilots should be used to inform the derivation of meaningful attribute descriptions.

After specifying the attributes of the healthcare service under valuation (i.e. risk of developing glaucoma, progressing to severe glaucoma, risk of becoming visually impaired, experiencing unwanted effects of treatment, the degree of communication and understanding with the healthcare provider), levels need to be assigned to them (e.g. 2, 6, 10, 15, 25 per 10,000 OHT individuals in 10-year time for the risk of becoming visually impaired). Qualitative attributes may be characterised by their nature (e.g. the local optician or hospital eye service in Box 10.1). When defining quantitative characteristics, a balance needs to be maintained between a larger number of levels, which results in more precise estimates, and increased complexity. A large number of attributes may also result in a design that is too large (e.g. too many choice tasks) and complex (e.g. too many attributes and alternatives) (de Bekker-Grob et al. 2012).

For our case study, the views of an advisory panel consisting of service users and experienced NHS healthcare professionals from primary and secondary care NHS eye services within the UK were sought. These were used to develop a framework (topic guide) for focus group discussions with service users (individuals with OHT). Pictorial cards were developed to illustrate common diagnostic test procedures, and the 10-year glaucoma risk was derived from treatment effectiveness data. The discussions were audiotaped, transcribed and analysed, and key themes from the focus group discussions were further developed using previous work developing a glaucoma utility index to capture the patients' perspective of the impact of glaucoma. Levels for each attribute were informed by focus group discussions, the literature, an existing economic model, the existing glaucoma utility measure and previous work on patient's communication and understanding.

10.4.2 Stage 2: DCE Choice Tasks (Generating an Experimental Design)

A crucial aspect of constructing a DCE is defining the choice sets. A full factorial design elicits preferences for all possible combinations of attributes and levels. This often results in a large number, and experimental design methods are used to create smaller fractional factorial designs. When employing full factorial designs, the researcher selects a set of choices (experimental design) which enable the main effects (i.e. the effect of each independent variable on the dependent variable) and possible interactions (i.e. preferences for one attribute depend on the level of another) to be estimated. Orthogonal designs are based on orthogonal arrays from design catalogues (e.g. Hahn and Shapiro 1966) or websites (Sloane 2009). These arrays have the properties of orthogonality (attributes that are statistically independent of one another) and level balance (levels of attributes appear an equal number of times). More recently, statistically efficient designs have been developed, with

statistical efficiency often defined in terms of D-efficiency (minimising determinant of the covariance matrix). SAS software generates such designs based on a generalised linear model (Kuhfeld 2000), while Ngene provides more flexible options (Choice-metrics 2016). For more on experimental design, see Johnson et al. (2013).

Our case study had 6 attributes with 4 levels and 1 attribute with 2 levels, resulting in 8,192 possible scenarios ($4^6 \times 2^1$). To reduce the number of possible combinations, a main-effects D-efficient design was generated, and estimates from the pilot data analysis were used (as priors) to generate the final design. This resulted in 32 choice sets.

After creating a DCE design, an important question is if an opt-out/current situation alternative should be included (e.g. 'no monitoring service' alternative in Fig. 10.1). The inclusion of an opt-out allows respondents to state that they prefer neither of the options, while its exclusion forces a choice. Forcing a choice may result in WTP being overestimated. However, opting out may be unrealistic in some choice situations (e.g. women's preferences for birthplaces). As a rule of thumb, analysts should define the choice scenario such that it represents the real-life choice. Given it is possible to opt out of an OHT monitoring programme, an opt-out was included in our case study. It is important to note that the levels for the attributes of the opt-out must be defined — in our case study, these were specified from the available literature and in consultation with the experts in the field. In other contexts, the opt-out may take on zero values or be individual specific and collected in the DCE.

10.4.3 Stage 3: Developing a Questionnaire

Having defined the DCE choice sets, analysts need to develop a questionnaire for data collection. Preferences may depend on individuals' characteristics. Thus, analysts need to formulate behavioural hypotheses about characteristics (e.g. gender, treatment history, severity of disease) that may affect valuation of healthcare experience. This information should be collected in the DCE questionnaire. In our case study, we collected information on income to test the economic prediction that households with a larger budget are willing to pay more. We also asked respondents to state their age, to test if this was a predictor of preferences (age 50 years generally marks the onset of presbyopia — need for reading glasses).

A well-designed questionnaire should reduce sources of potential survey-related issues such as non-responses, measurement error or a low response rate. Piloting the questionnaire will be important. Further information on characteristics of a good questionnaire are presented by Dillman et al. (2014). In our case study, the questionnaire was pre-piloted using an opportunistic sample (members of the research department), and a pilot study was conducted amongst 183 target respondents (taken from the same sample as the main survey).

10.4.4 Stage 4: Data Sampling and Analysis

10.4.4.1 Data Sampling

The sampling process should consider who to recruit to respond to the survey, how to administer the survey and sample size. Given decisions are being made about the allocation of resources provided by the general population, it may be argued that their views count. However, it may also be argued that within the context of healthcare, patients can make more informed choices. For our case study, while patient views were used to develop the DCE (attributes and levels), in the final survey, the views of the general population were elicited.

Most commonly DCE surveys were administered as postal questionnaires (de Bekker-Grob et al. 2012). However, the Internet now provides an alternative survey administration option, with several advantages compared to postal surveys: lower data collection costs, increased data collection speed, inclusion of multimedia elements and removal of manual data entry errors (Dillman et al. 2014). However, care should be taken to ensure representative samples and to check for 'speedsters' (individuals who complete the survey quickly). For our case, an online panel was used to collect data. Study representativeness was checked with appropriate quotas according to age, gender and location of the respondent. The response times for the whole survey as well as each question were retrieved in order to detect 'speedsters'. Then, respondents with consistent response times below 2 seconds per question were rejected.

A decision needs to be made about how many individuals should be enrolled in the DCE. There is little consensus on how to do this. The optimal number of participants depends on a variety of aspects, including the number of treatment options considered, the model to be estimated, the type and number of attributes and levels, potential interest in subpopulations as well as the perceived level of complexity of the DCE (Rose and Bliemer 2013). For more on sample size, see de Bekker-Grob et al. (2015).

10.4.4.2 Data Analysis and Interpretation

Analysis of DCE data is based on random utility maximisation (RUM) (McFadden 1974). RUM represents preferences by a utility function that takes higher values for more desirable alternatives. While respondents' utility is assumed to be deterministic, it cannot be observed by analysts. The utility function is defined as:

$$u_{in} = v_{in} + \varepsilon_{in} \tag{10.1}$$

where u_{in} is the overall utility that respondent n derives from alternative i, v_{in} is a function of the attributes, and defined levels, of the alternative and ε_{in} is the random

error. The deterministic part v_{in} of the utility is commonly assumed to be a linear function of the attributes and levels, such that:

$$v_{in} = \alpha_0 + \beta_1 \text{attribute\_1}_{in} + \beta_2 \text{attribute\_2}_{in} + \ldots + \beta_k \text{attribute\_k}_{in} \quad (10.2)$$

α_0 is referred to as the alternative specific constant, often picking up the preference of having treatment over no treatment (if there is an opt-out option). β_1 to β_k denote preference parameters (marginal utilities) that capture respondents' sensitivity to changes in the DCE attributes 1 to k (for given levels).

Table 10.1 presents the results for the estimated model for our case study (n = 814). The positive constant indicates a positive preference for monitoring. Respondents were less likely to choose a monitoring programme with a higher risk of conversion to glaucoma, severe glaucoma and visual impairment and unwanted side effects (indicated by the negative coefficients). Improving 'communication and understanding' and a hospital setting increased desirability of the monitoring programme (indicated by the positive coefficients).

The magnitude of the estimated parameters indicates the relative importance of each attribute level for a unit change. It is thus important to consider the unit of measurement. For example, the parameter for a continuous attribute (such as *cost* in our case study) shows the marginal utility of a one unit (£) change in cost (−0.018). For a categorical (qualitative) attribute, e.g. 'communication and understanding', a move to 'felt at ease and understood' (from the reference) is valued more than a move to 'felt at ease but did not understand' (0.861 > 0.475). Estimated parameters have also been used to calculate attribute 'importance scores' (e.g. Zickafoose et al. 2015).

Table 10.1 Preferences for the monitoring for OHT

Attribute and level	Coefficient	Marginal WTP (£) [95% confidence interval]
Alternative specific constant (*compared to no monitoring*)	0.508***	27.57 [18.9 to 35.4]
10-year risk of (*for OHT individuals*):		
Conversion to glaucoma	−0.001***	−0.03 [−0.03 to −0.02]
Progressing to glaucoma severe	−0.001***	−0.06 [−0.09 to −0.03]
Becoming visually impaired	−0.012***	−0.65 [−0.90 to −0.44]
Unwanted treatment effects (*compared to none*)		
Some	−0.286***	−15.50 [−18.7 to −12.9]
Quite a lot	−0.620***	−33.63 [−39.2 to −28.5]
Severe	−1.094***	−59.36 [−68.3 to −51.5]
Communication and understanding (*compared to not feeling at ease and did not understand*)		
Felt at ease and understood	0.861***	46.75 [40.4 to 54.4]
Felt at ease but did not understand	0.475***	25.76 [22.3 to 29.9]
Did not feel at ease but understood	0.480***	26.07 [22.1 to 31.1]
Hospital setting (*compared to local optician*)	0.025*	1.35 [−0.53 to 3.20]
Cost per year	−0.018***	

***Significant at <1% level; *Significant at <10% level (Source: based on Tables 29–30 from Burr et al. 2012)

A common output is the marginal rate of substitution (MRS) between an attribute h and an attribute 1. The MRS is defined as:

$$\widehat{MRS}_{h,1} = \frac{\partial \hat{v}_{in} / \partial \hat{\beta}_h}{\partial \hat{v}_{in} / \partial \hat{\beta}_1} = \frac{\hat{\beta}_h}{\hat{\beta}_1} \tag{10.3}$$

The MRS represents the trade-offs that respondents make by quantifying how much of attribute 1 (e.g. cost) they are willing to give up to receive a unit increase of attribute h (e.g. 'felt at ease and understood'). Typical MRSs are:

- $\widehat{MRS}_{h,-cost} = \hat{\beta}_h / -\hat{\beta}_{cost}$ is the most commonly used MRS and denotes marginal WTP for a unit increase of attribute h (see Table 10.1). For example, for our case study, a shift from 'no' unwanted effect of treatment to 'some' would reduce WTP by £15.50 per year (see Table 10.1).
- $\widehat{MRS}_{h,-waiting\_time} = \hat{\beta}_h / -\hat{\beta}_{waiting\_time}$ is willingness to wait for a unit increase of attribute h.
- $\widehat{MRS}_{h,-risk} = \hat{\beta}_h / -\hat{\beta}_{risk}$ is willingness to trade risk for a unit increase of attribute h.

A continuous attribute must be included to estimate MRSs. Equation 10.2 can also be used to predict uptake/acceptability of services (Adams et al. 2015; Morgan et al. 2015).

The commonly used conditional logit model, used to estimate Eq. 10.2, makes three assumptions: (i) preferences are homogeneous; (ii) the error term in Eq. 10.1 is independent across choice tasks; and (iii) the ratio between choice probabilities of all pairs of alternatives is constant (independence of irrelevant alternatives). Several alternative choice models have been put forward that relax these restrictive assumptions, with the random parameter logit being most prominent. The random parameter logit assumes that marginal utilities (β_1 to β_k in Eq. 10.2) are randomly distributed in the target population and accounts for correlation in the data. For further information on statistical methods, see Hauber et al. (2016), and for the applications of these models to our case study, see Hernández (2016).

10.5 Future Challenges when Incorporating a DCE into HTA

While DCEs provide useful information within a HTA framework, a number of challenges remain. De Bekker-Grob et al. (2012) identify a number of methodological questions in the application of DCEs in health, with a key question being the external validity of the method, i.e. do individuals behave in reality as they state in hypothetical surveys? This remains an important area for future research. Another important issue is whether respondents satisfy the underlying assumptions of DCEs.

A key assumption is that respondents are able to consider and trade all attributes that are important to them. There is a concern that respondents adopt simplifying decision heuristics (e.g. ignore attributes) to reduce the complexity of choices. This challenges the validity of estimated MRSs.

Within the context of broadening the valuation space, typical attributes in the DCE literature have focused on describing healthcare processes (e.g. waiting time, involved healthcare professionals, length of appointments and the frequency of treatments). Such aspects have been referred to as 'process utility' (Ryan et al. 2014a). While consideration of process utility extends the valuation space beyond health outcomes, it may still be limited. Entwistle et al. (2012) conducted a literature review to understand what characteristics patients value when receiving healthcare, why they value such characteristics and what associations might exist between the dimensions identified. Using a critical interpretive synthesis approach (a form of literature review that aims to integrate the conceptual and/or theoretical insights from a potentially diverse set of studies to help develop understanding of a particular topic) and existing conceptual frameworks, a conceptual map of patient experience was developed. A simplified version is presented in Fig. 10.2.

A key aspect of these experience dimensions is that they emphasise the importance of enablement through healthcare. To date, most DCEs focus on the 'characteristics' and 'act' dimensions of the conceptual map, with little consideration given to 'enablement' (Ryan et al. 2014b). Future research should explore how to value all three experience dimensions.

Another challenge in the use of DCEs within the HTA appraisal process is the time involved in conducting a DCE. Many HTA appraisals rely on the review of existing evidence — that is, they are based on secondary data with limited time (and

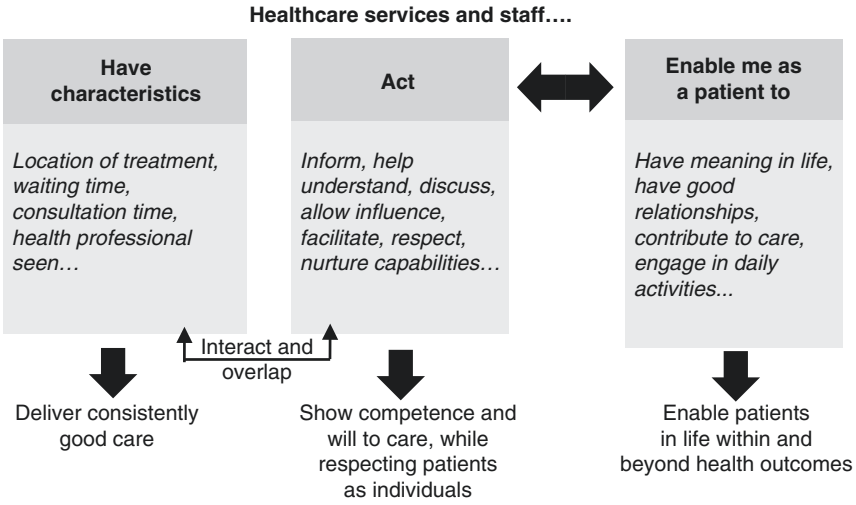

Fig. 10.2 Aspects of healthcare and healthcare professionals valued by patients

resources) to collect primary data. As such, it is unlikely that a DCE would be conducted as part of many appraisals. However, DCEs could be part of the evidence base submitted by a technology developer. Challenges on methods to synthesise these data can be expected and further research welcome.

Following on from the above point, DCEs are mostly (intentionally) specific to a particular healthcare decision. With often limited resources (time and money), this raises the question on how to apply DCE findings in a different decision-making context (known as benefit transfer). Within environmental economics, methods have been developed to transfer values from existing studies to new contexts (Johnston et al. 2015). Given there are now many published existing DCE studies, future research should explore the use of benefit transfer in a health context. Indeed, Bateman et al. (2002) argued that DCE methodology may be particularly well suited for benefit transfers.

10.6 Conclusion

DCEs provide potentially useful information in HTA: what characteristics (attributes) are important, how important, trade-offs between characteristics, monetary measures of value and predictions of take-up rates and acceptability of interventions/medicines/services. Important areas for research include: testing both the external validity of DCEs and whether individuals trade across attributes, what attributes should be included in the DCE (how is value defined), how to incorporate existing DCE results into evidence synthesis and generalisability of results from one DCE context to another (benefit transfer). Progress on these methodological issues will inform the HTA process, thus improving the delivery of patient and person-centred care.

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References

Adams J, Bateman B, Becker F, Cresswell T, Flynn D, McNaughton R, et al. Effectiveness and acceptability of parental financial incentives and quasi-mandatory schemes for increasing uptake of vaccinations in preschool children: systematic review, qualitative study and discrete choice experiment. Health Technol Assess. 2015;19:1–176. doi:10.3310/hta19940.

Australian Government, Department of Health. The pharmaceutical benefits advisory committee guidelines – appendix 7 utility valuation of health outcomes. 2014. https://pbac.pbs.gov.au/appendixes/appendix-7.html. Accessed 11 Sept 2016.

Bateman IJ, Carson RT, Day B, Hanemann M, Hanley N, Hett T, et al. Economic evaluation with stated preference techniques: a manual. Cheltenham: Edward Elgar Publishing Limited; 2002.

Bridges JFP, Hauber AB, Marshall D, Lloyd A, Prosser LA, Regier DA, et al. Conjoint analysis applications in health—a checklist: a report of the ISPOR good research practices for conjoint analysis task force. Value Health. 2011;14:403–13. doi:10.1016/j.jval.2010.11.013.

Burr J, Botello-Pinzon P, Takwoingi Y, Hernández R, Vazquez-Montez M, Elders A, et al. Surveillance for ocular hypertension: evidence synthesis and economic evaluation. Health Technol Assess. 2012;16:1–271. doi:10.3310/hta16290.

Choice-metrics. 2016. Ngene software. http://www.choice-metrics.com/index.html. Accessed 8 Sep 2016.

de Bekker-Grob EW, Ryan M, Gerard K. Discrete choice experiments in health economics: a review of the literature. Health Econ. 2012;21:145–72. doi:10.1002/hec.1697.

de Bekker-Grob EW, Donkers B, Jonker MF, Stolk EA. Sample size requirements for discrete-choice experiments in healthcare: a practical guide. Patient. 2015;8:373–84. doi:10.1007/s40271-015-0118-z.

Dillman DA, Smyth JD, Christian LM. Internet, phone, mail and mixed-mode surveys – the tailored design method. 4th ed. New Jersey: Wiley; 2014.

Entwistle V, Firnigl D, Ryan M, Francis J, Kinghorn P. Which experiences of healthcare matter to service users and why? A critical interpretive synthesis and conceptual map. J Health Serv Res Policy. 2012;17:70–8. doi:10.1258/jhsrp.2011.011029.

FDA (Food and Drug Administration). Patient preference information – voluntary submission, review in PMAs, HDE applications, and De Novo Re-quests, and Inclusion in decision summaries and device labeling: guidance for industry, food and drug administration staff, and other stakeholders. 2016. http://www.fda.gov/downloads/MedicalDevices/DeviceRegulationandGuidance/GuidanceDocuments/UCM446680.pdf. Accessed 2 Sept 2016.

Hahn G, Shapiro S. A catalogue and computer program for the design and analysis of orthogonal symmetric and asymmetric fractional factorial experiments. General Electric Research and Development Center: Schenectady, NY; 1966.

Hauber AB, González JM, Groothuis-Oudshoom CGM, Prior T, Marshall DA, Cunningham C, et al. Statistical methods for the analysis of discrete choice experiments: a report of the ISPOR conjoint analysis good research practices task force. Value Health. 2016;19:300–15. doi:10.1016/j.jval.2016.04.004.

Hernández R. Broadening the Valuation Space in Health Technology Assessment: the case of monitoring individuals with ocular hypertension. PhD thesis. University of Aberdeen, Scotland, UK, 2016.

IMI2-PROTECT. Patient perspective elicitation on benefits and risks of medicinal products, from development through the entire life cycle, to inform the decision-making process by regulators and Health Technology Assessment bodies. IMI2–2015–05–01. 2015. https://www.imi.europa.eu/content/imi-2-call-5-0 Accessed 6 Sept 2016.

IQWiG. Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen: Allgemeine Methoden Version 4.2. 2015. https://www.iqwig.de/download/IQWiG_Methoden_Version_4-2.pdf. Accessed 9 Sept 2016.

Johnson FR, Lancsar E, Marshall D, Kilambi V, Muehlbacher A, Regier DA, et al. Constructing experimental designs for discrete-choice experiments: report of the ISPOR conjoint analysis experimental design good research practices task force. Value Health. 2013;16:3–13. doi:10.1016/j.jval.2012.08.2223.

Johnston RJ, Rolfe J, Rosenberger RS, Brouwer R. Benefit transfer of environmental and resource values: a guide for researchers and practitioners. Dordrecht: Springer; 2015.

Kuhfeld W. Marketing research methods in the SAS system. 8th ed. North Carolina: The SAS Institute; 2000.

Lancsar E, Louviere J. Conducting discrete choice experiments to inform healthcare decision making. PharmacoEconomics. 2008;26:661–77.

Mccormack K, Wake B, Perez J, Fraser C, Cook J, Mcintosh E, et al. Laparoscopic surgery for inguinal hernia repair: systematic review of effectiveness and economic evaluation. Health Technol Assess. 2005;9 doi:10.3310/hta9140.

McFadden D. Conditional logit analysis of qualitative choice behavior. In: Zarembka P, editor. Frontiers in econometrics. New York: Academic Press; 1974. p. 105–42.

McIntosh E. Using discrete choice experiments within a cost–benefit analysis framework: some considerations. PharmacoEconomics. 2006;24:855–68.

Morgan H, Hoddinott P, Thomson G, Crossland N, Farrar S, Yi D, et al. Benefits of Incentives for Breastfeeding and Smoking cessation in pregnancy (BIBS): a mixed-methods study to inform trial design. Health Technol Assess. 2015;19 doi:10.3310/hta19300.

Mühlbacher AC, Bridges JF, Bethge S, Dintsios CM, Schwalm A, Gerber-Grote A, et al. Preferences for antiviral therapy of chronic hepatitis C: a discrete choice experiment. Eur J Health Econ. 2016; doi:10.1007/s10198-016-0763-8.

NICE (National Institute for Health and Clinical Excellence). CG85 glaucoma: diagnosis and management of chronic open angle glaucoma and ocular hypertension. London: Department of Health; 2009. https://www.nice.org.uk/guidance/cg85/resources/glaucoma-diagnosis-and-management-975690568645) Accessed 9 Sept 2016.

NICE (National Institute for Health and Care Excellence). Methods for the development of NICE public health guidance. third ed. London, UK: Department of Health; 2012. https://www.nice.org.uk/process/pmg4/chapter/incorporating-health-economics) Accessed 2 Sept 2016.

Robson SC, Kelly T, Howel D, Deverill M, Hewison J, Lie ML, et al. Randomised preference trial of medical versus surgical termination of pregnancy less than 14 weeks' gestation (TOPS). Health Technol Assess. 2009;13:1–124. doi:10.3310/hta13530.

Rose JM, Bliemer MCJ. Sample size requirements for stated choice experiments. Transport. 2013;40:1021–41.

Ryan M, Gerard K, Amaya-Amaya M. Using discrete choice to value health and health care. 1st ed. Dordrecht: Springer; 2008.

Ryan M, Gerard K. Inclusiveness in the health economics evaluation space. Soc Sci Med. 2014a;108:248–51. doi:10.1016/j.socscimed.2014a.01.035.

Ryan M, Kinghorn P, Entwistle VA, Francis JJ. Valuing patients' experiences of healthcare processes: towards broader applications of existing methods. Soc Sci Med. 2014b;106:194–203. doi:10.1016/j.socscimed.2014b.01.013.

Sloane N. A library of orthogonal arrays. 2009. www.research.att.com/~njas/oadir/ Accessed 13 Jul 2010.

Zickafoose JS, DeCamp LR, Prosser LA. Parents' preferences for enhanced access in the pediatric medical home: a discrete choice experiment. JAMA Pediatr. 2015;169:358–64. doi:10.1001/jamapediatrics.2014.3534.

Chapter 11
Analytic Hierarchy Process

Marion Danner and Andreas Gerber-Grote

11.1 Introduction

This chapter gives an overview on how the analytic hierarchy process (AHP) methodology can be used to elicit patients' preferences and presents case studies on how this methodology may inform HTA and HTA-based decisions. Patients' preferences are, together with external scientific knowledge and physician's experience, the tenets of evidence-based medicine (Sackett et al. 1996). To ensure that data on patients' preferences is considered as robust evidence for decision-making, it should be generated in a methodologically sound, structured, and transparent way. AHP is a multiple-criteria decision-analytic (MCDA) method that can be used to elicit patients' preferences for specific treatment characteristics or outcomes assessed in HTA. The steps in conducting an AHP are depicted. AHP follows transparent mathematical rules for data analysis but has its own methodological challenges and opportunities as depicted in this chapter. Examples of how AHP may be used in HTA and decision-making are provided and discussed.

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11.2 A Role for AHP in Patient Preference Elicitation

A number of recent reviews and pilot applications in HTA have pointed to the potential of MCDA methods to guide healthcare decision-making or get structured information on patient preferences (Marsh et al. 2014; Maruthur et al. 2015; Danner et al. 2011; Ho et al. 2015). The AHP, which was developed by the mathematician Thomas L. Saaty in the 1970s (Saaty 1977), is, among others, one MCDA approach that can be used to elicit preferences and measure the relative importance of decision criteria to decision-makers including patients (Hummel et al. 2014; Dolan 2010). Recent reviews (Adunlin et al. 2015a; Marsh et al. 2014) suggest AHP is the most used MCDA technique.

While DCE (Chap. 10) is rooted in expected utility theory, AHP is a decision-analytic approach. It decomposes and structures a decision problem into its basic elements, asks decision-makers to value these elements relative to each other, and then combines these judgments to generate composite value information on the alternatives or criteria making up a decision problem. AHP is not an approach to assess value in terms of money or utility units as is the DCE. Rather, it aims to assign a relative value to its elements—generating relative importance weights. Saaty proposed AHP to facilitate complex decision-making, especially for groups of decision-makers (Saaty 1977, 1994). According to Whitaker (Whitaker 2007, p. 859), the AHP in group decision processes "tends to give better results because of the broader knowledge available and also because of the possibility of debates that may arise and change people's understanding." While DCE is based on the assumption that patients try to maximize their utility each time they make a choice, AHP assumes no normative theory predicting choices but admits that individuals—in terms of bounded rationality—often deviate from the basic assumptions of rationality (Simon 1978; Kinoshita 2005). Rather the goal of AHP is to structure a decision and make values and preferences transparent to enable informed and—ideally—more rational decisions. This is why AHP is considered a "descriptive" rather than a "normative" theory.

In healthcare, AHP is often used to elicit preferences from experts (clinicians, administrators) to support structured and transparent decision or planning processes (Benaim et al. 2010). It has, to a lesser extent, been used to elicit preferences from patients, sometimes in larger samples (Dolan et al. 2013b; Kuruoglu et al. 2015) or limited to small patient group surveys (Danner et al. 2011). Eliciting preferences from patients instead of other decision-makers is different in several ways. Firstly, patients are direct consumers of the decisions they take regarding their own health. Decision-relevant criteria often cause anxieties and involve uncertainty or, e.g., risks of side effects, which impact decision heuristics. Secondly, information asymmetries dominate the decision situation. Physicians are usually well informed about the evidence and have professional experience, while the patient is less well informed and lacks professional experience. On the other hand, health illiteracy—especially about statistical information such as risks or probabilities—is prevalent on both sides, physician and patient, and adequate communication about these issues remains insufficient (Envisioning Health Care 2020, 2011).

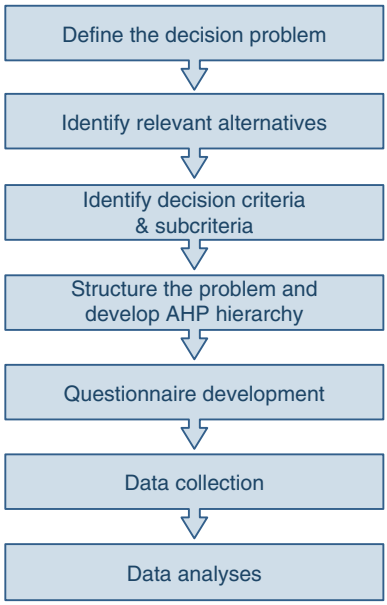

Fig. 11.1 Steps in developing an AHP

11.3 Steps in Developing an AHP Preference Elicitation

Similar to the recommendations published by the International Society for Pharmacoeconomics and Outcomes Research (ISPOR) for the development of a DCE study (Bridges et al. 2011; Johnson et al. 2013), an AHP should be carefully prepared and well designed, as depicted in Fig. 11.1.

In line with the ISPOR recommendations, a literature search, expert interviews, as well as qualitative work with patients should be employed to select and refine decision-relevant criteria and subcriteria.

11.4 How Does AHP Work?

The AHP allows elicitation of preferences using a procedure of pairwise comparison between decision criteria and treatment alternatives (Saaty 1977; Dolan 1989). AHP first structures a decision in a hierarchy (Fig. 11.2). The objective of a decision is positioned at the highest level (e.g., to weigh the benefits and risk of alternative treatments), followed by relevant decision criteria (e.g., effectiveness or side effects of alternative treatments) and clusters of subcriteria at the next level(s) (e.g., reduction of different symptoms to specify the effectiveness criterion). The treatment alternatives are placed at the lowest level of the AHP hierarchy but may not be part of the AHP preference elicitation process. Elements in the hierarchy should be comprehensive to give a complete picture of the decision situation. Lower-level

elements should be independent of the next higher-level elements, and elements at one level should ideally not overlap. Also, a comparison between two criteria should be independent of a third criterion at that level in the cluster.

AHP is either used in a more comprehensive assessment to prioritize or rank the performance of alternative treatment options based on preferences for decision criteria and alternatives or, in a more reduced form, to just elicit respondents' preferences for different decision criteria to measure their relative importance (Angelis, Kanavos 2016). The total number of pairwise comparisons at each level of the hierarchy and in each cluster of criteria is given by $(n^*(n-1))/2$ (see blue and green lines in Fig. 11.2). In these pairwise comparisons, AHP respondents express how strongly they prefer one criterion, subcriterion, or alternative compared to the other one. The strength of preference is usually measured on a two-sided nine-point ratio scale. While each point on the scale has an ordinal verbal interpretation to facilitate judgments, the numerical (ratio scale) values are used in AHP weight calculations (see Fig. 11.3).

When comparing criteria *i* and *j*, choosing "1" on the scale means that criterion *i* is equally important or preferred by the patient as criterion *j*, 3 means *i* is moderately more important than *j*, 5 means much more important, 7 means very much more important, and 9 means extremely more important. The intermediate values 2, 4, 6, and 8 may also be chosen. If alternatives are part of the procedure, the question

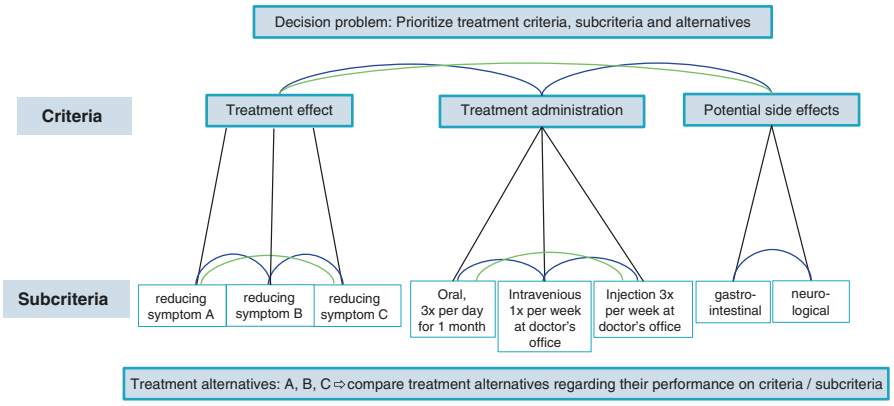

Fig. 11.2 Example AHP decision hierarchy and pairwise comparisons

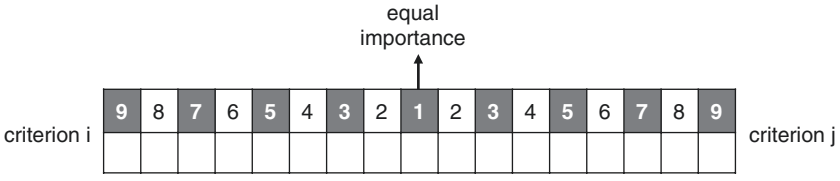

Fig. 11.3 AHP scale

format reflects how an alternative is rated on its performance relating to two specific (sub)criteria compared to each other.

Based on pairwise comparisons provided, the AHP uses a direct mathematical approach to calculate "importance" weights for each of the included criteria and alternatives. All comparisons resulting from the AHP survey are first transferred to a comparison matrix $A = [a_{ij}]$. Values at the upper right side of the matrix's diagonal are the result of actual pairwise comparisons; values at its lower left side are their reciprocals. Local importance weights in AHP are calculated using the principal right eigenvector approach (Saaty 1977; Dolan 1989), which represents the vector of weights (w) of included criteria/subcriteria in case of a reciprocal matrix. The calculation is based on the following matrix algebraic equation: some comparison matrix A multiplied by its right eigenvector w is, in case of a nonnegative reciprocal matrix, equal to the matrix's maximal eigenvalue λ_{max} multiplied by w ($A \times w = \lambda_{max} \times w$). Based on this relationship, the right eigenvector may be estimated for each matrix by, for example, using the matrix multiplication method (Dolan 1989). In practical terms, this process is "a simple averaging process by which the final weights are the average of all possible ways of comparing the scores on the pairwise comparisons" (Hummel et al. 2014). Alternative calculation and analysis modes may be used (Ishizaka, Labib 2009; Dolan 1989). AHP weight vector calculation may well be performed in Microsoft Excel but is also supported by professional software (e.g., Expert Choice Comparison, http://expertchoice.com/comparion/, or SuperDecisions, www.superdecisions.com). For a comprehensive list of available software, see Hummel et al. (2014).

Data aggregation for groups of AHP respondents may be done in two ways. Aggregated weights may be calculated as the average mean of individual weights calculated based on individual judgments (so-called aggregation of individual priorities (AIP)) or by calculating AHP weights for the group based on the geometric mean of all individual judgments (so-called aggregation of individual judgments (AIJ)) (Forman and Peniwati 1998). The aggregation method should depend on the specific decision context. If the group is considered as one entity striving for consensus, the AIJ aggregation is usually preferred. If the focus is on eliciting individual preferences of a group of heterogenous decision-makers, the AIP method is preferred. The latter might be more relevant in patient preference research where heterogenous groups of individuals are surveyed.

Finally, AHP allows calculation of a "consistency ratio (CR)," which measures the logical consistency of pairwise judgments within a cluster of judgments. The concept of consistency relies on two basic assumptions of the AHP: the transitivity of preferences (i.e., if $A > B$ (A preferred to B) and $B > C$, then $A > C$) and the reciprocity of judgments. While transitivity is a necessary condition for consistency, AHP does not require that preferences are perfectly transitive. Technically, the CR measures how much the measured consistency of a matrix, the consistency index (CI), differs from the average consistency (the so-called random index, RI) of a simulated set of reciprocal but totally random pairwise comparison matrices. The consistency index of matrix A is calculated by the following formula: $CI = \frac{\lambda_{max} - n}{n-1}$.

The CR is defined as CI/RI. The closer CI and RI are, the higher the CR and the greater the probability that judgments in a comparison matrix result from a completely random decision process. The smaller the CI in relation to the RI, the smaller the CR and the higher the probability that judgments are the result of a consistent decision process. For details on calculation of the consistency ratio and its components, refer to the literature (Saaty 2000; Dolan et al. 1989).

11.5 Using AHP to Elicit Patients' Preferences in Healthcare Policy and HTA

Awareness is increasing that the views and preferences of patients as primary consumers of health interventions should be taken into account at various levels and steps in decision-making. Involving patients early in HTA and decision processes may increase the legitimacy of final decisions. Knowing patient preferences can further help determine which health technologies, interventions, and types of services should be offered to patients. It can also increase adherence to them. Technically, AHP has the advantage of facilitating direct calculation of preference weights for individual patients, which is beneficial if AHP is to be integrated in decision aids, for example. Aggregated preference information, on the other hand, can feed into decisions to prioritize interventions at the other decision levels. Since a current overview of AHP applications can be found in Schmidt et al. (2015) (Schmidt et al. 2015) and Adunlin et al. (2015b) (Adunlin et al. 2015b), a sample of recent publications was used to demonstrate how AHP may enable the uptake of patient preference information into HTA-based decision processes.

11.5.1 Example 1: Health Policy Decisions—Uptake of Preventive Screening Measures

Three recent AHPs elicited patient preferences for colorectal cancer screening interventions in different settings and using different survey modes (Xu et al. 2015; Hummel et al. 2013; Dolan et al. 2013a, b). Two studies were conducted in the USA and one in the Netherlands. They were administered as paper-pencil questionnaire (Xu et al. 2015) and online survey (Hummel et al. 2013) or in personal interviews (Dolan et al. 2013a, b). While Xu et al. (2015) limited their study population to individuals who had experienced screening before, such restrictions were not applied in the other studies. All studies concluded that patient preference information is indispensable, especially in helping to understand why certain screening programs have better uptake than others.

11.5.1.1 Study Findings and Insights

All studies identified clinical outcome criteria such as the "prevention of cancer" or the "sensitivity" or "accuracy" of the screening method to detect cancer, as well as the "safety/complication frequency/side effects" of the test as being most relevant. The studies also suggest that process-related screening characteristics such as the frequency of the test or the complexity of test preparation (or "convenience of test" or "logistics") play an important role when patients finally decide to undergo a test or not. Therefore, when offering a specific screening test to a population, it is important to adequately inform patients about test characteristics to ensure uptake.

11.5.1.2 Methodological Insights

Only 167 of the 650 patients (26%) who returned the completed questionnaire in the AHP study by Hummel (Hummel et al. 2013) provided consistent responses—based on a consistency ratio below 0.3. Dolan (Dolan et al. 2013a, b) included 379 of 484 (78%) of patients and Xu (Xu et al. 2015) included 667 out of 954 (70%) patients with a consistency ratio below 0.15 in their analyses. Including inconsistent respondents in AHP analyses might bias study results. Excluding them, on the other hand, might put the external validity of a study at stake. AHP studies should explore the effects of excluding inconsistent respondents on results in sensitivity analyses as in Hummel et al. In addition, the demographic or disease-related characteristics of included participants may be compared to the overall target population to explore reasons for inconsistency. Technical reasons for inconsistency that were identified by the authors were the complexity and a large number of pairwise comparisons and not providing the option to patients to revise inconsistent judgments. While Xu et al. indicate that due to the individuality of patient preferences, the aggregation of patient priorities was performed using the described AIP method, the other studies do not explicitly provide information on the chosen aggregation mode.

11.5.2 Example 2: Health Policy Decisions—Drug Reimbursement or Approval

The German Institute for Quality and Efficiency in Healthcare (IQWiG) regularly assesses the additional benefit of new drugs seeking reimbursement (Chap. 25). These HTAs focus on clinical outcomes measuring mortality, morbidity, side effects, and quality of life. IQWiG conducted two preference elicitation studies where patients valued the importance of treatment outcomes in different indications to test whether this information could be used to prioritize outcome-specific HTA results. One of these studies was conducted using AHP (Danner et al. 2013; Gerber-Grote et al. 2014). Another DCE study that identified patient preferences

for outcomes and characteristics of lung cancer treatments was recently submitted by a pharmaceutical company to support the benefit assessment of the lung cancer drug afatinib (Muhlbacher and Bethge 2015; Gemeinsamer Bundesausschuss (G-BA) 2014).

11.5.2.1 Study Findings and Insights

The IQWiG AHP study was conducted in a group setting, separately with patients and clinicians (Danner et al. 2013). Patients valued treatment outcomes differently from clinicians. They considered fast response to treatment most important, while experts considered remission and avoidance of relapse most important. Patients also rated the quality of life dimensions cognitive function, reduction of anxiety, and social function higher than experts. The DCE lung cancer study (Muhlbacher and Bethge 2015) found that the clinical treatment endpoints of progression-free survival and reduction of the tumor-specific symptoms such as coughing, shortness of breath, and pain were most relevant and of comparable importance to patients.

11.5.2.2 Methodological Insights

The IQWiG pilot projects suggest that AHP or other preference methods may be used to generate weights or prioritize outcome-specific HTA results. Yet, no gold standard method for preference elicitation exists, and methods like AHP or DCE will need further research and testing in practical applications to learn more about their specific characteristics and suitability in different settings. Using patients as the target population in these assessments seems legitimate since their preferences as "consumers" of healthcare interventions likely differ from their physicians', the general public, or other HTA stakeholders' preferences (Muhlbacher and Juhnke 2013; Danner et al. 2011). The German AHP study further points to the potential of group studies in patients since the group setting facilitates exchange of information and experience. Group interaction is also likely to help increase the consistency of judgments by avoiding judgmental errors or misunderstandings. Group studies may, on the other hand, suffer from dominant individuals' leading group discussions (Thokala et al. 2016). In the DCE lung cancer study, patient preferences were elicited including progression-free survival as surrogate endpoint for overall survival as one outcome. IQWiG in the respective hearing stated that—in its view and in accordance with many other regulatory and HTA bodies—the (most) important patient-relevant endpoint overall survival was not included in the preference elicitation task (Gemeinsamer Bundesausschuss (G-BA) 2014). It is thus important to select outcomes or criteria for preference elicitation which are accepted within a specific HTA decision context.

Elicitation of patients' relative judgments may be an important tool to inform and support authorities' outcome-specific evidence or benefit/risk prioritization

preceding approval or reimbursement decisions. The latter was also highlighted by the FDA in its recently released guidance on patient preference research (FDA Center for Devices and Radiological Health 2016).

11.6 Where and How Should Information on Patient Preferences Inform HTA?

As the examples above suggest, AHP may be used at different levels in decision-making. While a range of stated preference or multi-criteria-analytic methods may be used to elicit preferences, methods like DCE or AHP have the advantage to force patients to make trade-offs between criteria. An advantage of AHP could be its ability to directly calculate weights for individual decision criteria—in contrast to DCE, where this is only indirectly possible using attribute-level ranges and, therefore, dependent on the chosen levels. AHP is less easily applicable to generate utilities or exchange rates (e.g., in monetary units) compared to a DCE and may not be readily usable in cost-utility analyses to support resource allocation. A study by Reddy et al. (2015), however, takes up the AHP as an alternative to time-trade-off to calculate utilities for EQ-5D health states based on ordinal preference data from an AHP. The authors conclude that the described method "… offers the potential to convert ordinal preference data into cardinal utilities" being "simpler than TTO *(time trade off)* studies to carry out…." Whether AHP might in the future play a role in such applications remains to be seen; DCEs or other conjoint analytic techniques are currently preferred in these instances.

11.7 Practical and Methodological Issues with AHP

In line with most recent ISPOR recommendations, an AHP as any MCDA study should be carefully developed and follow the steps only recently suggested by the respective MCDA task force (Thokala et al. 2016; Marsh et al. 2016). Careful selection and refinement of decision criteria, the combination of quantitative with qualitative elements, and a transparent documentation and calculation of importance weights are essential (Marsh et al. 2016). The practical aspects of an AHP, such as survey format or administration, depend on the target population and the study objective. An AHP group setting or an interviewer-assisted questionnaire administration both may facilitate the generation of combined qualitative and quantitative information on patient preferences. In the group setting, patients provide judgments, then discuss the individual judgments in a group, and finally may revise their individual judgments. While the group setting might suffer from influential or dominant participants, the qualitative component provides insights into patients' reasoning and their decision-making processes. This may equally be attained in an interviewer-assisted setting if patients are asked to think aloud throughout providing judgments. Individual

online or paper-pencil surveys, on the other hand, are limited to quantitative information. Also, inconsistency might be higher. Striking a balance between internal consistency (excluding inconsistent respondents) and external validity (including all respondents) is a challenge. An option to reduce inconsistency would be to use online tools, such as those offered by the Expert Choice software, asking participants to verify their judgments in case of high inconsistency. Researchers will have to define which setting (group or individual, interviewer-assisted or not, online or in-person) and which type of information (qualitative and/or quantitative) are needed.

In the literature, inconsistency ratio thresholds from 0.1 to 0.3 have been used to identify inconsistent respondents. While in a group setting a CR of 0.1 might be a good threshold, it is likely not for large individual surveys, where a limit of 0.2 or 0.3 seems reasonable. There is no agreement on the thresholds to use in preference studies yet.

The face validity of an AHP study may be increased by following rigorous steps in the development of the design (e.g., comprehensive set of relevant criteria, ensure independence of criteria at one level) and by using qualitative elements to verify the generated quantitative information (Marsh et al. 2016). Also, convergent validity could be tested by assessing preferences using different methods for preference elicitation. There is some debate about the reliability of AHP in that interviewing the same group of patients at different points in time might lead to different findings. This is likely true since preferences depend on patient characteristics and patient characteristics change over time. Hence, a clear definition of the study population is important.

AHP has undergone a range of methodological criticisms. Probably the issue that has been most frequently raised in the past years is rank reversal. This may be observed in AHP and other MCDA methods when an identical copy of an alternative or new but non-discriminating criterion is added to the decision hierarchy (Maleki and Zahir 2013). Several methodological recommendations (e.g., comprehensiveness of AHP hierarchy, relevance of included criteria) and analysis modes (e.g., ideal eigenvector standardization mode) to prevent or minimize the risk of rank reversal have been proposed and are frequently applied (e.g., Wang and Elhag 2006; Hummel et al. 2014; Ishizaka, Labib 2009). Other issues are the appropriateness of the AHP judgment scale and the search for other/more appropriate scales to reflect respondents' values. Several publications discuss the AHP ratio scale and its potential limitations (e.g., not a continuous scale, being bounded, or not appropriately representing verbal judgments) (e.g., Dong et al. 2008; Hummel et al. 2014). Other scales avoiding the potential weaknesses of the AHP scale were developed (e.g., Lootsma or geometric scales, other continuous, smaller, or wider scales) and are explored, but the AHP scale is still the most used scale. Finally, the relatively "abstract" pairwise comparison of individual criteria in AHP—while making the procedure easy and transparent—has been criticized. In comparison, a DCE presenting entire choice sets to respondents appears to be a more realistic and holistic approach and easier to understand for patients who are used to choose between treatment alternatives. However, most recent AHP studies suggest that AHP is feasible for patients once they understand the type of task—including the AHP judgment scale—they have to perform (Danner et al. 2016).

11.8 Conclusion

As for any method to be used in HTA or other kinds of health-related assessments, AHP is not suited for all kinds of decision-making in healthcare. We consider AHP a very valuable preference elicitation tool, especially to enrich decision aids or prioritize criteria, endpoints, or alternatives in complex preference-sensitive and HTA-based decision-making. However, we also caution its application. There are methodological challenges, so it is important to present these and account for them transparently (e.g., in sensitivity analyses). It should be noted that while there is no "gold standard" approach for patient preference elicitation, research is always a dynamic and ongoing process.

References

Adunlin G, Diaby V, Montero AJ, Xiao H. Multicriteria decision analysis in oncology. Health Expect. 2015a;18:1812–26. doi:10.1111/hex.12178.

Adunlin G, Diaby V, Xiao H. Application of multicriteria decision analysis in health care: a systematic review and bibliometric analysis. Health Expect. 2015b;18(6):1894–905. doi:10.1111/hex.12287.

Angelis A, Kanavos P. Value-based assessment of new medical technologies: towards a robust methodological framework for the application of multiple criteria decision analysis in the context of health technology assessment. PharmacoEconomics. 2016;34(5):435–46. doi:10.1007/s40273-015-0370-z.

Benaim C, Perennou DA, Pelissier JY, Daures JP. Using an analytical hierarchy process (AHP) for weighting items of a measurement scale: a pilot study. Rev Epidemiol Sante Publique. 2010;58:59–63. doi:10.1016/j.respe.2009.09.004.

Envisioning Health Care 2020. Better doctors, better patients, better decisions. Cambridge MA: MIT Press; 2011.

Bridges JF, Hauber AB, Marshall D, Lloyd A, Prosser LA, Regier DA, et al. Conjoint analysis applications in health—a checklist: a report of the ISPOR good research practices for conjoint analysis task force. Value Health. 2011;14:403–13. doi:10.1016/j.jval.2010.11.013.

FDA Center for Devices and Radiological Health. Patient Preference Information—Voluntary Submission, Review in Premarket Approval Applications, Humanitarian Device Exemption Applications, and De Novo Requests, and Inclusion in Decision Summaries and Device Labeling. Guidance for Industry, Food and Drug Administration Staff, and Other Stakeholders. http://www.fda.gov/downloads/%20MedicalDevices/DeviceRegulationandGuidance/GuidanceDocuments/UCM446680.pdf (2016). Accessed 29 Aug 2016.

Danner M, Gerber-Grote A, Volz F, Wiegard B, Hummel JM, Ijzerman MJ et al. Analytic Hierarchy Process (AHP)—Pilotprojekt zur Erhebung von Patientenpräferenzen in der Indikation Depression. 2013. https://www.iqwig.de/download/Arbeitspapier_Analytic-Hierarchy-Process_Pilotprojekt.pdf. Accessed: 11 Nov 2016.

Danner M, Hummel JM, Volz F, van Manen JG, Wiegard B, Dintsios CM, et al. Integrating patients' views into health technology assessment: analytic hierarchy process (AHP) as a method to elicit patient preferences. Int J Technol Assess Health Care. 2011;27:369–75. doi:10.1017/S0266462311000523.

Danner M, Vennedey V, Hiligsmann M, Fauser S, Gross C, Stock S. How well can analytic hierarchy process be used to elicit individual preferences? Insights from a survey in patients suffering from age-related macular degeneration. Patient. 2016;9(5):481–92. doi:10.1007/s40271-016-0179-7.

Dolan JG. Medical decision making using the analytic hierarchy process: choice of initial antimicrobial therapy for acute pyelonephritis. Med Decis Mak. 1989;9:51–6.

Dolan JG. Multi-criteria clinical decision support: a primer on the use of multiple criteria decision making methods to promote evidence-based, patient-centered healthcare. Patient. 2010;3:229–48. doi:10.2165/11539470-000000000-00000.

Dolan JG, Boohaker E, Allison J, Imperiale TF. Can streamlined multicriteria decision analysis be used to implement shared decision making for colorectal cancer screening? Med Decis Mak. 2013a;34:746–55. doi:10.1177/0272989X13513338.

Dolan JG, Boohaker E, Allison J, Imperiale TF. Patients' preferences and priorities regarding colorectal cancer screening. Med Decis Mak. 2013b;33:59–70. doi:10.1177/0272989X12453502.

Dolan JG, Isselhardt Jr BJ, Cappuccio JD. The analytic hierarchy process in medical decision making: a tutorial. Med Decis Mak. 1989;9:40–50.

Dong Y, Xu Y, Li H, Dai M. A comparative study of the numerical scales and the prioritization methods in AHP. Eur J Oper Res. 2008;186:229–42. doi:10.1016/j.ejor.2007.01.044.

Forman E, Peniwati K. Aggregating individual judgements and priorities with the analytic hierarchy process. Eur J Oper Res. 1998;108 doi:10.1016/S0377-2217(97)00244-0.

Mündliche Anhörung gemäß 5. Kapitel § 19 Abs. 2 Verfahrensordnung des Gemeinsamen Bundesausschusses—hier: Wirkstoff Afatinib [database on the Internet]. 2014. Available from: https://www.g-ba.de/downloads/91-1031-87/2014-03-25_Wortprotokoll_end_Afatinib.pdf. Accessed: 6 Oct 2016.

Gerber-Grote A, Dintsios CM, Scheibler F, Schwalm A, Wiegard B, Mühlbacher A et al. Wahlbasierte Conjoint-Analyse—Pilotprojekt zur Identifikation, Gewichtung und Priorisierung multipler Attribute in der Indikation Hepatitis C. 2014.

Ho MP, Gonzalez JM, Lerner HP, Neuland CY, Whang JM, McMurry-Heath M, et al. Incorporating patient-preference evidence into regulatory decision making. Surg Endosc. 2015;29:2984–93. doi:10.1007/s00464-014-4044-2.

Hummel JM, Bridges JF, IJzerman MJ. Group decision making with the analytic hierarchy process in benefit-risk assessment: a tutorial. Patient. 2014;7:129–40.

Hummel JM, Steuten LG, Groothuis-Oudshoorn CJ, Mulder N, Ijzerman MJ. Preferences for colorectal cancer screening techniques and intention to attend: a multi-criteria decision analysis. Appl Health Econ and Health Policy. 2013;11:499–507. doi:10.1007/s40258-013-0051-z.

Ishizaka A, Labib A. Analytic hierarchy process and expert choice: benefits and limitations. OR Insight. 2009;22:201–20.

Johnson RF, Lancsar E, Marshall D, Kilambi V, Muhlbacher A, Regier DA, et al. Constructing experimental designs for discrete-choice experiments: report of the ISPOR conjoint analysis experimental design good research practices task force. Value Health. 2013;16:3–13. doi:10.1016/j.jval.2012.08.2223.

Kinoshita E. Why we need AHP/ANP instead of utility theory in today's complex world—AHP from the perspective of bounded rationality. 2005. http://citeseerx.ist.psu.edu/viewdoc/download;jsessionid=C1A22937FCE9669436D827A37E0A1689?doi=10.1.1.333.5543&rep=rep1&type=pdf. Accessed: 6 Oct 2016.

Kuruoglu E, Guldal D, Mevsim V, Gunvar T. Which family physician should I choose? The analytic hierarchy process approach for ranking of criteria in the selection of a family physician. BMC Med Inform Decis Mak. 2015;15:63. doi:10.1186/s12911-015-0183-1.

Maleki H, Zahir S. A comprehensive literature review of the rank reversal phenomenon in the analytic hierarchy process. J Multi-Criteria Decis Anal. 2013;20:141–55. doi:10.1002/mcda.1479.

Marsh K, Lanitis T, Neasham D, Orfanos P, Caro J. Assessing the value of healthcare interventions using multi-criteria decision analysis: a review of the literature. PharmacoEconomics. 2014;32:345–65. doi:10.1007/s40273-014-0135-0.

Marsh K, IJzerman M, Thokala P, Baltussen R, Boysen M, Kalo Z, et al. Multiple criteria decision analysis for health care decision making-emerging good practices: report 2 of the ISPOR MCDA emerging good practices task force. Value Health. 2016;19:125–37. doi:10.1016/j.jval.2015.12.016.

Maruthur NM, Joy SM, Dolan JG, Shihab HM, Singh S. Use of the analytic hierarchy process for medication decision-making in type 2 diabetes. PLoS One. 2015;10:e0126625. doi:10.1371/journal.pone.0126625.

Muhlbacher AC, Bethge S. Patients' preferences: a discrete-choice experiment for treatment of non-small-cell lung cancer. The Europ J Health Econ. 2015;16(6):657–70. doi:10.1007/s10198-014-0622-4.

Muhlbacher AC, Juhnke C. Patient preferences versus physicians' judgement: does it make a difference in healthcare decision making? Appl Health Econ Health Policy. 2013;11:163–80. doi:10.1007/s40258-013-0023-3.

Reddy BP, Adams R, Walsh C, Barry M, Kind P. Using the analytic hierarchy process to derive health state utilities from ordinal preference data. Value Health. 2015;18:841–5. doi:10.1016/j.jval.2015.05.010.

Saaty TL. A scaling method for priorities in hierarchical structures. J Math Psychol. 1977;15 doi:10.1016/0022-2496(77)90033-5.

Saaty TL. Highlights and critical points in the theory and application of the analytic hierarchy process. Eur J Oper Res. 1994;74 doi:10.1016/0377-2217(94)90222-4.

Saaty TL. Fundamentals of decision making and priority theory with the analytic hierarchy process. Pittsburgh PA: RWS Publications; 2000.

Sackett DL, Rosenberg WM, Gray J, Haynes RB, Richardson WS. Evidence based medicine: what it is and what it isn't. BMJ. 1996;312:71.

Schmidt K, Aumann I, Hollander I, Damm K, von der Schulenburg JM. Applying the analytic hierarchy process in healthcare research: a systematic literature review and evaluation of reporting. BMC Med Inform Decis Mak. 2015;15(1):112. doi:10.1186/s12911-015-0234-7.

Simon HA. Rationality as process and as product of thought. Am Econ Rev. 1978;68:1–16.

Thokala P, Devlin N, Marsh K, Baltussen R, Boysen M, Kalo Z, et al. Multiple criteria decision analysis for health care decision making-an introduction: report 1 of the ISPOR MCDA emerging good practices task force. Value Health. 2016;19:1–13. doi:10.1016/j.jval.2015.12.003.

Wang Y-M, Elhag TMS. An approach to avoiding rank reversal in AHP. Decis Support Syst. 2006;42:1474–80. doi:10.1016/j.dss.2005.12.002.

Whitaker R. Validation examples of the analytic hierarchy process and analytic network process. Math Comput Model. 2007;46:840–59. doi:10.1016/j.mcm.2007.03.018.

Xu Y, Levy BT, Daly JM, Bergus GR, Dunkelberg JC. Comparison of patient preferences for fecal immunochemical test or colonoscopy using the analytic hierarchy process. BMC Health Serv Res. 2015;15:175. doi:10.1186/s12913-015-0841-0.

Chapter 12
Ethnographic Fieldwork

Tine Tjørnhøj-Thomsen and Helle Ploug Hansen

12.1 Introduction

The aim of the chapter is to introduce the readers to ethnographic fieldwork including participant observation and ethnographic interviews. Ethnographic fieldwork is a robust research methodology to study patients' experiences and perspectives and, therefore, particularly valuable for HTA. Furthermore, it leads to important insights that are relevant to patient involvement in HTA. The chapter is divided into five sections. In the first section, we briefly introduce ethnographic research in relation to patient involvement in HTA. In the following three sections, we focus first on fieldwork, then on participant observation and then on ethnographic interviewing. In the next two sections, we address field notes as an important research activity in ethnographic fieldwork and consider analytical work. We close the chapter discussing what we consider to be important dimensions of knowledge production. First, we invite critical reflection on the notion of patient involvement and the increasing popularisation of ethnographic and qualitative methodology. Then we briefly address ethnography in familiar settings, and finally we discuss the importance of transparency and reflexivity in knowledge production.

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12.2 Ethnographic Research Design

Patient involvement in HTA invokes many questions related to research designs, methodology and research methods. In this chapter, we focus on ethnographic fieldwork, which is a specific research design developed in anthropology to explore the dynamic relationship between social worlds and cultural systems and how social actors think, act and interact. Within anthropology and related disciplines and epistemologies, there is a long tradition of observing and participating in the informants' everyday life, talking to and listening to what they say, thereby gaining knowledge about the informants' (patients, consumers, caregivers, etc.) perspectives and experiences as well as their activities, social interactions and relationships. Thus, from an anthropological perspective, it is unthinkable *not* to involve the people under study and to be involved with them in the process of knowledge production. We will address this further throughout the chapter.

To our knowledge, there are only a few HTA reports that have used ethnographic fieldwork as their research design. One Danish HTA assessed the ward rounds at a Danish Hospital as a social practice and the implications of the ward round for the different actors. The methods used were observational studies, focus group interviews, individual interviews and time registration (Willemann et al. 2006). Another Danish HTA investigated how patient involvement in relation to heart rehabilitation and arthritis treatment could be shaped and developed. The empirical study took place in units for heart rehabilitation and arthritis treatment, where the researcher observed a number of consultations and conducted qualitative interviews with patients, nurses and doctors (Jacobsen et al. 2008).

Conducting ethnographic fieldwork requires that the researcher joins the people under study where they live or work for a period of time to observe and experience their everyday life and grasp their point of view (Malinowski 1961). Knowledge is thus achieved by sharing space and time for a period with the people or the community that constitutes the field of investigation. Ethnographic fieldwork encompasses a range of research methods, the principal being participant observation and ethnographic interviews. We address these methods as we consider them highly relevant and useful in relation to HTA. Ethnographic fieldwork is usually time-consuming. Often the researcher stays with people under study for a longer period of time (3 or 6 months or even longer). This is seldom possible in an HTA. However, a shorter field study (e.g. one to 3 weeks) may provide insights and knowledge of vital importance for the HTA.

12.3 Fieldwork

Ethnographic fieldwork includes a range of methods primarily participant observation and interviews. According to the article 'Patients Perspectives in HTA: A Route to Robust Evidence and Fair Liberation' by Facey et al. (2010), the most commonly used qualitative methods 'for generating evidence to determine patient's

perspectives are individual in-depth interviews and focus group' (ibid., p. 37). The authors emphasise that participant observation can 'supplement' interviews (ibid.). Firstly, because what people *say they do* and what they *actually do* can be contradictory, and secondly, because participant observation is useful for an understanding of the physical, social, cultural and economic context in which patients live or are receiving care. In this section, we elaborate on these important points. We consider ethnographic fieldwork, participant observation and ethnographic interviews as crucial for an understanding of the social world and the social activities in which people in particular situations engage (Atkinson 2015, p. 4).

In relation to HTA, fieldwork can be highly relevant in order to understand the social world of the patients, for instance, how they perceive and act in relation to health technologies such as screening, home dialyses versus hospital dialysis, ostomy bags or patient education. A small fieldwork study based on participant observation can do much more than just supplement different forms of interviews. We suggest that in the production of an HTA, patient involvement should not depend on or prioritise one method like interviews or recorded talk (Atkinson 2015, p. 38). Fieldwork and participant observation are unique in that the empirical material arises through the researchers' involvement with the people under study (Tjørnhøj-Thomsen and Whyte 2008, p. 92). Even though a patient may have valuable experiential knowledge about a specific illness, condition or health technology that can be explored in an individual interview, knowledge and perspectives are not only told, they are also acted out in social interaction and social context. When, for instance, people use a blood pressure monitor to check their blood pressure at home, they may operate it in ways that adjust to their everyday life routines and may not therefore follow the doctor's instruction. In addition, some patients may be taciturn or not responsive to interview techniques. Hence, we cannot obtain patients' perspectives only by interviewing them, even though interviews like all forms of conversations are important for getting patients' perspectives, attitudes, belief and expectations about health, illness and health technologies (Facey et al. 2010, p. 234). Since patients' perspectives both influence and are influenced by social relationships, it is crucial also to understand how patients and their close relatives act and interact in different situations.

12.4 Participant Observation

Participant observation is the primary methodological practice of ethnographic fieldwork. The researcher takes part in people's lives and observes them through sharing (some) time and social space. Participant observation (often described as an *oxymoron*) implies two forms of simultaneous research activities. Where the activity *observation* implies distance and a more passive stance from the researcher, the activity *participation* implies involvement with the people and activities under study. Participant observation means that the researcher performs both these activities in turn and to different degrees. Participant observation is thus a methodological

strategy that the researcher uses in order to actively take part in, experience, observe and record the lives of the participants under study (Spradley 1980; Tjørnhøj-Thomsen and Whyte 2008). The epistemological idea is that by being there and taking part in people's lives, the researcher gains an opportunity to experience and understand what the social world looks like and how the context (the ward, the hospital, the home, the organisational division of labour, the technology) frames how people think and act (ibid., Atkinson 2015).

12.4.1 Access and Gatekeepers

A methodological and ethical problem for all researchers is that of recruiting and gaining access to relevant research participants and, in the case of ethnography, to acquire a place among them in this space or community. The problem of access is not definitively solved, by gaining permission from formal gatekeepers. Permission from, for instance, a hospital administrator or consultant to follow patients through investigations and treatment is a necessary but far from sufficient condition for including patients or clinical staff in a study. Patients, caregivers and healthcare professionals are also the gatekeepers of their own lives, meaning that they also always pick out what they want to tell the researcher. Furthermore, permissions do not automatically entitle the researcher access to different actors' activities and viewpoints. Negotiating access is an ongoing activity, where the researcher must continuously explain the purpose of the study and the reasons for his/her presence (Tjørnhøj-Thomsen and Whyte 2008, p. 98 ff). Access is thus an integral part of the social relations and forms of exchange that an HTA researcher enters into in order to gain empirical insight into the specific health technology (Tjørnhøj-Thomsen 2003). Furthermore, it may also provide knowledge about the subtle processes through which specific spaces are not available for study. In some instances, it is the researcher's own boundaries that hold her back; in other cases, it is those of others. People may or may not be willing to be involved, or they may be sceptical and reserved towards the study. Thus, negotiating access or involvement also contributes to the empirical material. Researchers can gain important insights from recording and reflecting on the motives that, for instance, patients may have for participating in an HTA as well as from the barriers they set up, because these motives and barriers may contain important information about the technology under assessment (ibid.). Negotiating access is therefore an important way of gaining information about the gatekeepers' and boundary setters' perspectives and concerns and forms part of the empirical material. Getting access also implies obtaining informed consent. Within ethnography, informed consent is an interactive process. It requires the HTA researcher to inform potential participants of the purpose and procedures of the research. Furthermore, the risks and benefits associated with the study and how the data provided by the participant will be protected and stored must also be communicated. Within ethnographic fieldwork, consent must often be negotiated in the course of the study, and it is not always possible to document the consent in a

written form, e.g. if the informants are illiterate or feel uncomfortable signing a written document (American Anthropological Association 2016).

12.4.2 Everyday Life and Context

Participant observation has the advantage that it provides knowledge about patients' everyday life with a specific health technology, which they may take for granted in everyday routine situations and, therefore, forget to talk about in interviews or do not find relevant. Participant observation is also particularly well suited to investigate relations and interaction between people—as well as the interaction between people and their physical, material or institutional context (Spradley 1980; Atkinson 2015; Tjørnhøj-Thomsen and Whyte 2008, p. 94). Thus, if the researcher only wishes to obtain knowledge of how patients (or their caregivers and relatives) verbally express their experience of staying in a hospital ward, live with a rare disease and use a new telehealth app or a new medicine, interviews or focus group discussions may be a relevant choice of methods. When, however, researchers want to understand how the patients, caregivers or relatives act and live with a specific condition and manage different health technologies, including telehealth solutions, in their daily lives, contextual dimensions become important (Hansen et al. 2011). Contextual dimensions can include the place/setting, the specific technical equipment and mode of delivery, used in the health technology (such as medical devices, apps, monitors, screening apparats), as well as the activities and daily routines, clinical encounters and what people do and say in this space. However, what counts as context is also a choice that the researcher makes, meaning that researchers contextualise in order to give form to their interpretation (Dilley 1999, p. 1). Participant observation is also useful for studying how patients are involved in the HTA process.

12.4.3 Social Situations

The specific knowledge potential and performance of participant observation depends on the specific HTA and the social situation connected to this. According to James Spradley, all participant observation takes place in social situations. Therefore, the first step in doing ethnography is to locate a social situation (or situations) that is relevant for the study and accessible for the researcher (Spradley 1980). In an HTA, such a situation could, for instance, be a patient in home dialysis or patients in a rehabilitation programme. Spradley defines a social situation as a situation bounded by *place*, *actors* and *activities*. Spradley's notion of situation (ibid.) may serve as a guide for engaging in participant observation. *Space* relates to the physical place or places (including physical surroundings, social atmosphere, objects, furniture and decor). *Actors* relate to the people involved in the

situation (who are present) and *activity* to a set of related acts (e.g. communication, health professionals supporting patients with daily hygiene and shared decision-making) people do. Applying these three aspects of a situation to participant observation in an HTA also emphasises the importance of not only defining the relevant situation for the HTA, but also of following the informants in and through different social situations. Thereby, the researchers can learn how the patients' experiences, views, perceptions and preferences change with situation, context and time. Participant observation thus refers to a strategic effort to take part in, observe and systematically register people's lives on their own premises in different social situations. What the researchers observe, sense and thereby come to know (Wolcott 1994) depends, therefore, on the nature of their participation, the place they are allotted in the empirical context and the social situations and activities they engage in.

12.4.4 Observations

Participant observation can of course focus more on observation than on participation or vice versa, depending on the specific HTA or research project. Even though observation may be the main activity of the researcher, he or she is always also and simultaneously a participating actor (whose participation also needs to be observed and documented through careful note-taking.) Qualitative and ethnographic researchers point out that little attention had been given to the 'fine art of observation' (Tjora 2006, p. 431) and that conducting observations of good scientific quality requires training and systematics. Therefore, we want to pay closer attention to *observation*. Observation methods are useful because they enable the researcher to explore what people actually do (and forget to tell the researcher about), social interaction and communication (who interacts and communicates with whom) and to register how much time is spent on various activities (Kawulich 2005). What to observe always (like other data generating methods) depends on the aim of the study. Observation is a fundamental method in producing knowledge and sound scientific research to inform HTA about patients' experiences. Observation does not mean merely to *watch*, *see* or *look* at something—but also includes other senses such as smell, taste, hearing and touch. It nevertheless requires methodological reflections about from where, what and how the researcher observes (or senses), and the researcher's aims (Wolcott 1994). For that reason, the preparation of an observation guide may be just as important as preparing an interview guide. Such a guide can support the researcher to strategise carefully about what to observe, with what purpose and from where (activities, surroundings, symbols, social interactions, who-does-what, etc.) The researcher may, for example, first apply an *open* observation strategy to provide a broad-spectrum insight into what is going on in a particular health technology setting and the characteristics of the setting. Thereafter, one may apply a more focused strategy by observing particular social situations including the interplay between places, actors and activities (ibid.).

12.5 Interviews

In the production of an HTA, it may be of great relevance to interview patients, relatives, health professionals and other stakeholders. Interviewing is an important research method in exploring how patients articulate experiences, perspectives, attitudes, needs and desires in relation to a specific health technology (Chap. 4). Conducting an interview is—as participant observation—closely related to the researcher's research interest and scientific background, including his or her philosophical, epistemological and theoretical approach. Whether the researcher conducts the interview as a 'face to face' individual interview, a focus group interview or using telephone or Skype, chatroom, Facebook or questionnaire interviews (when compared to informal conversations), the HTA researchers always has a specific purpose. In order to perform an interview, the researcher may use semi-structured guides; conversational techniques around a theme, a picture, a photograph or a video; or open unstructured interviews, which may take the form of a sickness or life history interview (Spradley 1979, Denzin and Lincoln 2011). Interviews may last for a short period of time, for instance, 10–20 min, or they may last for hours (Jeppesen et al. 2015). In an ethnographic study, an interview can also be seen as participant observation, and there is not a clear-cut boundary between the two methods. In the course of the fieldwork and while spending time with the informants, the researcher engages in informal and formal talk with the informants about what they are doing and thinking. The researcher both observes and takes part in and (more or less) guides the interview and notes down details about the social situation of the interview and context, including the atmosphere, the setting, the actors and the activities. Interviews are forms of social interaction, where the patient and the HTA researcher both engage bodily in the dialogue. Therefore, the researcher needs to observe simultaneously since the digital recorder only captures the conversational exchange (Tjørnhøj-Thomsen and Whyte 2008, p. 107). And conversely, participant observation does not exclude conversations and interviewing. Rather, it is part and parcel of any conversation. In the following we focus on the ethnographic interview as a specific kind of qualitative interview.

12.5.1 Ethnographic Interviewing

An ethnographic interview can be understood as a speech event, sharing features with a friendly conversation. Skilled ethnographers often generate most of their empirical material through participant observation and casual conversations while perhaps introducing a few ethnographic questions (Spradley 1979, p. 58). Although we may understand an ethnographic interview as a conversation, sharing some of the characteristics of other forms of everyday conversation, the ethnographic interview has an *explicit purpose* coming from the researcher. As a speech act, it has its own specific agenda, where, for instance, repetitions and asking questions are an integrated part (Spradley 1979, pp. 55–57).

12.5.1.1 Ethnographic Questions

In order to maintain focus in the interview, it may be relevant to create an interview guide. We suggest that such a guide is based on Spradley's description of the ethnographic interview (ibid., p. 67). In relation to interviewing in HTA, we suggest the list may include the following components.

Components in the Ethnographic Interview

1. Greetings and establishing a friendly and balanced relationship with the patient
2. Explaining the purpose of the interview and the agenda
3. Providing ethnographic explanations
4. Raising descriptive questions (Spradley 1979)

12.5.1.2 Descriptive Questions

Spradley divides descriptive questions into five major types of questions: *grand tour*, *mini tour*, *example questions*, *experience questions* and *native-language questions* (ibid., pp. 86–87). In HTA with a focus on patient involvement, we suggest that the first four types of questions are relevant.

12.5.1.3 Grand Tour Questions

Typically, grand tour questions ask the person to generalise, including patterns of events, typical situations and typical subjects. These questions often start with phrases such as 'Could you describe …', for example, 'Could you describe a typical day at home using your telehealth app?' or 'Could you tell me about how you usually contact the nurse' (ibid., p. 87).

12.5.1.4 Mini Tour Questions

When the patient engages in answering, commenting on and discussing grand tour questions, a richness of description will often open up for an in-depth investigation of more detailed aspects of experience (ibid., p. 88). If the HTA researcher, for instance, asked the patient during the grand tour 'Could you describe a typical day at home using your telehealth app', this could lead to a mini tour question, such as 'Could you describe how you used your telehealth app yesterday?'. Grand tour and mini tour questions are in many ways similar to each other. The difference is that mini tour deals with smaller units of experience than grand tour questions.

12.5.1.5 Example Questions

In some HTAs the grand and mini tour questions will be sufficient. However, using phrases such as 'Could you give me an example of what you do if your telehealth app does not work?' may often be very helpful to make the patients tell more and even narrate their answers.

12.5.1.6 Experience Questions

In most HTAs concerned with patient involvement, the researcher or the patient will be interested in talking about their experiences with a treatment, a new device, symptoms, etc. The researcher may, for instance, open such a dialogue by saying 'Could you describe how you feel about using the telehealth app at home four times per day?' or 'Could you tell me about how you experienced the information session about the app at the outdoor clinic?'

Summarising, the ethnographic interview always depends on contextual dimensions, such as the interview setting, the behaviour of the interviewer, the responses from the interviewee, the interview length and the positions and roles undertaken by the interviewer and the interviewee. Furthermore, it is important to consider that it is perhaps through the interview the patient becomes aware of his/her emotions, views, needs and life challenges and puts them into words. Therefore, the knowledge produced through ethnographic or other kinds of interview is co-constructed knowledge based on and through the researcher's intervention.

12.5.2 Recording of an Interview

The researcher can choose between a range of techniques to record the interviews. In most cases the interview will be recorded in a digital form. This way of preserving an interview gives the researcher an opportunity to engage in the actual interview situation with full attention. Furthermore, it is important that the researcher also take field notes (see the next section).

12.6 Field Notes

In the course of fieldwork with participant observation and ethnographic interviews, experience and observation are recorded and stored as *field notes*. Field notes are the researchers' thick descriptions of social situations including description of places, actors and activities and a record of what people say and do. Field notes are thus part of the empirical material that subsequently becomes the object of analysis, and they often provide good cases or illustrations for the analytic points in the HTA

assessment report (Tjørnhøj-Thomsen and Whyte 2008, p. 109). It is not always possible to take comprehensive notes during the participant observation or interview situations. Often the researcher may only have time and space for scratch notes or writing down keywords and memos. However, later it is important to write a full and coherent account of the situation. Taking notes is also an important tool for critical reflection. When rereading the notes, the researcher may 'discover that there are aspects that she must ask about in greater depth at a later point' (ibid., p. 112), or the notes may spark ideas for theoretical and analytical work. It may also be useful to distinguish between three forms of notetaking (Bernard 1994):

(1) *Descriptive notes*, where the researcher makes thorough and uninterpreted descriptions of the setting and the people under study, including what people do and say. These notes are used in making 'thick descriptions'.
(2) *Methodological notes*, in which the researcher registers and reflects on whether the chosen methods prove relevant or useful in the HTA setting or not, and in what ways and why they can be adjusted in the course of the study. These notes may be part of the empirical material (HTA material); they may enhance methodological reflections and knowledge and help to create transparency and validity.
(3) *Analytic notes*, which concern analytical concepts and theoretical ideas or perspectives that emerge during the fieldwork or interview and may be used in the ongoing analysis.

12.7 Considerations on the Analytic Process

In ethnography and qualitative methods, the analysis begins during the fieldwork (Atkinson 2015, pp. 14–15). It is, for example, through taking and reading field notes that ideas for the analysis and sense-making surface. Thus, in ethnographic research, analysis and data collection are interlinked. The analytical work requires that the researcher reads through the material (transcribed interviews and field notes) several times to get an overall impression of what is at stake, identifies the predominant themes and becomes familiar with the diversity of the empirical material. The researcher engages the empirical material by sorting, organising and coding it with the purpose of developing and conceptualising more general cross-cutting themes. This is often referred to as a *thematic analysis* in which you link, compare and contrast themes across the ethnographic material. The identification of themes is driven by the research or HTA questions and theoretical interests, but in the process it is crucial to include unexpected or contradictory themes and findings, make comparisons between findings from other studies and bring them in dialogue with relevant theories. Writing is an integral part of the analytical work beginning with transcribing interviews and writing down field notes to create a coherent text or story. In the process, the researcher tries to relate themes to one another and develop general statements, new concepts and analytical points, most often in dialogue with theoretical perspectives (Emerson et al. 1995). As we mention below, the analytical work is—as part of the knowledge production—reductive and selective. It is also a process of generalisation, for instance, by

identifying and conceptualising some general principle concerning the phenomenon in question (Sharp 1998). This means that the generalisation that is a result of an ethnographic study or other kinds of qualitative studies is analytical or theoretical and not empirical (ibid.). The analytical generalisation implies that the findings from a particular study may be relevant or transferable to other contexts.

12.8 Discussion: The Production of Knowledge

Patient involvement as the concept is defined in this book (Chap. 1) is, as we have shown, an unavoidable and integrated part of ethnographic fieldwork. Patients are persons with particular experiences of illness and health technologies and experts in their own particular life situation. Patients' expertise on their own lived experiences is crucial in research and HTAs. This particular understanding of involvement does not, however, free the researcher from having to take a critical and reflexive discussion about the notion of patient involvement. It is, for instance, important to keep in mind that patients do not form a homogenous group and may have diverging interests and positions including as experts in their disease or representatives of a disease, a handicap or a disability. Therefore, patient involvement implies questions such as: What is meant by the notion of patient? Who involves whom and for what reason and under which circumstances? What are the implications for the knowledge production of the actual forms of involvement? (Chap. 3).

Furthermore, when reflecting on the notion of patient involvement, for instance, regarding patient participation in the HTA process, it may be important to consider the differences between patients' and researchers' contributions to HTA and thus the character of patient involvement in research (Chaps. 4, 5, and 8). We raise this issue, because we sometimes meet the assumption that conducting interviews and participant observation does not require special training and expertise compared to, e.g. statistical or epidemiological research. The increasing popularisation of ethnographic and qualitative methodology combined with the drive for patient involvement may result in a de-valuing of what a researcher brings to the field. Research, however, implies training in systematic literature review, generating and collecting data, critical analyses, theory-based interpretations and ethical and presentational considerations. Researchers may of course use their personal experiences (also) as patient in HTAs, but the training as researcher includes (besides theoretical and methodological knowledge) critical reflexivity in relation to research activities, i.e. how experience, positioning, roles, identity, perspectives and interests may influence the knowledge production (Chaps. 3 and 8).

12.8.1 Ethnography in Familiar Settings

Anthropologists have for long reflected on and disputed the challenges that may arise when the anthropologist is studying part of the society, where he or she belongs. This discussion is far beyond the scope of this chapter as it is closely related to what has been termed the 'repatriation of anthropology', meaning that

anthropologist increasingly have turned to study their own societies (Amit 2000). It is, however, worth mentioning one important point in this debate. Researchers working in settings and among people or issues with which they are familiar or have prior knowledge need to be attentive not to take the familiar for granted, because they think they know what is at stake. Thus, there is a risk that researchers who are familiar with the setting forget, neglect or avoid to ask questions to what seem obvious, thereby leaving cultural or setting-specific notions implicit. It may, therefore, be useful to position oneself as a potential not-knower and continuously interrogate one's own as well as the informant's knowledge.

12.8.2 Transparency and Reflexivity

This final section discusses transparency and reflexivity in knowledge production (Hastrup 2004). In particular, we draw attention to three aspects: critical conceptual work, relational dimensions and the selective and reductive characteristics of knowledge.

First, it is important to reflect on the use and meaning of concepts (ibid). When a researcher, for instance, chooses to use the concept of patient *preferences*, it means something different from choosing to use the concept of patient *experiences*. The concept of patient preferences carries meaning connected to how patients decide between different options of, for example, treatment, screening and medicine (Chaps. 11 and 25), whereas the concept of *patient experiences* carries meaning connected to views, thoughts and perceptions of the disease or the use of the health technology. It is a concept far more diffuse in definition and meaning than patient preferences. This means that the knowledge produced in an HTA always also reflects the meanings inherent in the concepts used, whether it is patient preferences or patient experiences or something else (Chap. 3). Therefore, concepts, categories, terms and words have a constituting impact on the knowledge product (Hastrup 2004). Also, the notion of technology needs to be defined and described in an HTA, since not only cultural perceptions of technology but also the nature of the particular technology (e.g. whether it is medical equipment or rehabilitation) under assessment will influence the design and choice of methods used.

A second and important aspect is the *relational dimensions* of knowledge production. The Danish anthropologist Kirsten Hastrup states that knowledge 'has become - and must be—acknowledged (implicitly, at least) as *relational*, both in the sense that it attaches itself to relations between people or between people and objects and in the sense that it emerges within a dialogical field' (Hastrup 2004, p. 456, original italics). The researcher gains knowledge, Hastrup suggests, by engaging in social relations in the field (among the people under study) as well as in the academic institution. That is the reason why knowledge can never be objective in the classical meaning of the concept; it is always partial, positioned and situated (Harraway 1988).

Thirdly, Hastrup also makes another important point relevant for primary research, HTA and knowledge production in general. She suggests that knowledge is always both reductive and selective: 'It is reductive because it renders empirical complexity and messiness in clear, but therefore also more limited, propositions about the world. It is selective, because for it to be knowledge it has to disregard some information' (Hastrup 2004, p. 256). A critical reflection and discussion of the knowledge produced in HTA about, for instance, patients' perspectives and experiences needs therefore to address the reducing and selecting activities embedded in the knowledge production. If, for example, a patient representative is participating in an HTA, it is not enough that the patient representative makes reflections, the researchers must reflect on to what extent and how the knowledge gained from this individual representative represents a larger group of patients' experiences with the technology. The researchers must of course also reflect critically on the other stakeholder's perspectives in the course of knowledge production. In Chap. 3, Hansen and Street discuss some of the different terms used in relation to patient involvement in HTA including patient as experts, patient advocates or representatives of a disease and patients as consumers. The point is that each term incorporates assumptions about different patient roles that may be played out individually or in groups. It is, therefore, important that the HTA researchers continuously raise a critical and reflective stance towards the knowledge produced through, for instance, ethnographic fieldwork, participant observation and ethnographic interviews. This requires that the HTA researchers develop the necessary skills for undertaking ethnographic fieldwork.

12.9 Concluding Remarks

In this chapter, we have demonstrated how ethnographic fieldwork can provide robust scientific evidence that provides important insights into patients' experiences of their illness or use of a health technology that may not be apparent from other forms of research and so could contribute important new knowledge to inform HTA. The ethnographic fieldwork design, participant observation and ethnographic interviews are well-established, robust and solid research strategies and highly relevant for different types of assessments of health technology: *firstly*, because ethnographic fieldwork including participant observation and ethnographic interviews challenge the dominance and the methodological limitations of only conducting semi-structured interviews in obtaining patients' experiences and perspectives; *secondly*, because ethnographic fieldwork optimises the possibilities for ethically sound involvement of the patients; and *thirdly*, because ethnographic fieldwork can provide nuanced, comprehensive and scientific insights to improve HTAs. It is crucial to pay attention to how patients (and their caregivers) act, react and interpret technologies. To explore patients' perspectives is thus not to identify one 'true' perspective through an individual interview. Patients' perspectives are emerging, relational and shifting. Therefore, there is a need for enhancing methodological and

epistemological reflections and discussions about future development of ethnographic fieldwork in relation to HTA and patient involvement. The potential use of ethnographic fieldwork including participant observation and ethnographic interviews will be highly relevant in relation to the assessment of new screening procedures, telehealth solutions and collaboration between different sectors such as hospital, municipalities and general practice. Furthermore, ethnographic fieldwork would be of importance for exploring how technology is working in local settings.

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References

American Anthropological Association. http://www.americananthro.org/ParticipateAndAdvocate/Content.aspx?ItemNumber=1652- (2016). Accessed 3 Sept 2016.

Amit V. Constructing the field. Ethnographic fieldwork in the contemporary world. London: Routledge; 2000.

Atkinson P. For ethnography. Thousand Oaks: Sage Publications; 2015.

Bernard HR. Research methods in anthropology. Qualitative and quantitative approaches. London: Sage Publications; 1994.

Denzin NK, Lincoln YS. The sage handbook of qualitative research. 4th ed. London: Sage Publications; 2011.

Dilley R. The problem of context. New York: Berghahn Books; 1999.

Emerson RM, Fretz RI, Shaw LL. Writing ethnographic fieldnotes. Chicago: The University of Chicago Press; 1995.

Facey K, Boivin A, Gracia J, et al. Patients' perspectives in HTA: a route to robust evidence and fair liberation. Int J Technol Assess Health Care. 2010;26:34–40.

Hansen HP, Tjørnhøj-Thomsen T, Johansen C. Rehabilitation interventions for cancer survivors: the influence of context. Acta Oncol. 2011;50:259–64.

Harraway D. Situated knowledges. The science question in feminism and the privilege of partial perspective. Fem Stud. 1988;14:575–99.

Hastrup K. Getting it right: knowledge and evidence in anthropology. Anthropol Theory. 2004;4:455–72.

Jacobsen CB, Pedersen VH, Albeck K. Patientinddragelse mellem ideal og virkelighed - en empirisk undersøgelse af fælles beslutningstagning, og dagligdagens møder mellem patient og behandler. København: Sundhedsstyrelsen, Monitorering & Medicinsk Teknologivurdering; 2008, Medicinsk Teknologivurdering—puljeprojekter 2008; 8(3)/DSI Rapport 2008.07.

Jeppesen J, Rahbek J, Gredal O, Hansen HP. How narrative journalistic stories can communicate the individual's challenges of daily living with amyotrophic lateral sclerosis. Patient. 2015;8:41–9.

Kawulich BB. Participant observation as a data collection method. Forum: Qualitative Social Research. 2005; 6 Art. p. 43.

Malinowski B. Argonauts of the Western Pacific. New York: E.P Dutton; 1961.

Sharp K. The case for case studies in nursing research: the problem of generalization. J Adv Nurs. 1998;27:785–9.

Spradley J. The ethnographic interview. New York: Holt, Rinehart and Winston, Inc; 1979.

Spradley J. Participant observation. New York: Holt, Rinehart and Winston, Inc; 1980.

Tjora AH. Writing small discoveries: an exploration of fresh observers' observations. Qual Res. 2006;6:429–51. doi:10.1177/1468794106068012.

Tjørnhøj-Thomsen T. Samværet. Tilblivelser I tid og rum [Intercourse, Becomings in Time and Space]. In: Hastrup K, editor. Ind I verden. En grundbog I antropologisk metode [Into the World. An Introduction to Anthropological Method]. Copenhagen: Hans Reitzels Forlag; 2003. p. 93–115.

Tjørnhøj-Thomsen T, Whyte S. Fieldwork and participant observation. In: Vallgårda S, Koch L, editors. Research methods in public health. Copenhagen: Munksgaard; 2008. p. 91–119.

Willemann M, Svendsen MN, Ankjær-Jensen A, Petersen PG, Christensen M. Stuegang—en medicinsk teknologivurdering med fokus på vidensproduktion, Medicinsk Teknologivurdering—puljeprojekter 2006; 6(1)/DSI Rapport 2006.02. København: Sundhedsstyrelsen, Center for Evaluering og Medicinsk Teknologivurdering; 2006.

Wolcott HF. Confession of a "trained" observer. In:Transforming qualitative data. Description, analysis and interpretation. Thousands Oaks: Sage; 1994.

Chapter 13
Deliberative Methods to Involve Patients in HTA

Jackie M. Street and Edilene Lopes

13.1 Introduction

This chapter argues for the use of deliberative methods to involve patients in HTA. Deliberation refers to 'communication that induces reflection on preferences, values and interests in a non-coercive fashion' (Mansbridge et al. 2010, p. 2). Deliberative democracy or deliberative governance posits the notion that 'policy making requires spaces where different institutions, agencies, groups, activists and individual citizens can come together to deliberate on pressing social issues' (Hendriks 2009, p. 173). In HTA, deliberative spaces already exist in patient organisations and in government advisory panels which include experts and in some cases citizen or patient 'representatives'. However, many patients still feel disenfranchised from systems which often appear to value clinical efficacy and cost-effectiveness over patient wellbeing and which often marginalise patient voices (Lopes et al. 2016). This chapter describes methods which aim to be *inclusive* of patients' preferences, values and interests by bringing a diverse group of patients and/or patient representatives into informed deliberation to directly influence the policy process. This approach differs from collection of patient views and experience through qualitative approaches in that patients act as active participants in the policy deliberation. The chapter first introduces some elements of good deliberation and deliberative democratic theory that are pertinent to the argument and presents some involvement methods based on this theory. Current and potential involvement of stakeholders in HTA deliberation is mapped. The methodological and philosophical elements are then examined for their potential applicability to patient involvement in HTA.

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13.2 Deliberative Inclusive Methods

A range of deliberative inclusive methods or 'mini-publics' have been developed in the last 50 years in response to shortcomings in the capacity for representative democracy to address the needs of disadvantaged and marginalised groups and in a parallel attempt to improve shortfalls in decision-makers' understanding of community values and perspectives (Ozanne et al. 2009). Goodin and Dryzek (2006) describe mini-publics as 'small group deliberations composed of ordinary citizens designed to be groups small enough to be genuinely deliberative and representative enough to be genuinely democratic'. Such methods are an attempt to operationalise the tenets and intent of deliberative democratic theory. Deliberative democracy, itself, is a recent development in democratic theory in opposition to a 'rational choice/aggregative' account of democracy (Bohman and Rehg 1997). Despite the diversity in the methods, scholars generally agree that all methods are designed to capture the views of multiple stakeholders in the decision-making through reasoned informed debate about policy options. Deliberative inclusive methods move beyond simple debate in that there are no winners or losers in deliberation and the overall aim is to arrive at a consensus decision, that is, the process is collaborative rather than adversarial (Paul et al. 2008; Hodgetts et al. 2014; Street et al. 2014).

Fishkin (Fishkin 2009) describes mass democracy (which generally is a rational choice/aggregative model) as public consultation on people's 'top of their head' opinions, whereas deliberative democracy would include discussion of topics among citizens under 'good' conditions. In this sense, deliberative democratic models, which employ these methods, are desirable because they allow people to consider policy issues in depth and in relation to both the evidence and the potential consequences to others rather than only from an individual viewpoint. In addition, deliberative inclusive methods, which support discussion of policy topics according to set rules, can act to ensure high-quality measured debate in the public realm. Consequently, used appropriately, deliberative inclusive methods can legitimise contentious policy decision-making (Bohman and Rehg 1997; Dryzek 2000; Fishkin 2009). Since health matters touch the lives of citizens in a very personal way, HTA frequently strays into areas of contentious policy decision-making where good deliberation can be essential for policy success.

13.2.1 Principles of Good Deliberation

Taking Fishkin's model (Fishkin 2009) as an example, quality deliberation requires several elements shown in Table 13.1.

Therefore, normative accounts of good deliberation dictate the inclusion of a descriptively representative and diverse range of voices from the affected population. That is, as far as possible, the participants should be chosen to reflect the range of opinions held by the broader population on the issue under discussion. In addition,

Table 13.1 Required elements for quality deliberation (Fishkin 2009)

Element	Description
Information	Access to reasonably accurate information relevant to the issue
Substantive balance	Arguments are offered in a balanced range of perspectives
Diversity	Major positions in the public represented by participants in the discussion
Conscientiousness	Participants sincerely weigh the merits of the arguments
Equal consideration	Where arguments offered by all participants are considered on the merits regardless of which participants offer them

the participants should be exposed to a broad view of the evidence which participants can challenge through interaction with experts. Both elements are important so that the participants are exposed to a range of views born of research-derived evidence and personal experience. The deliberation should be facilitated by an independent facilitator whose purpose is to ensure that all voices are heard, to strive for balanced evidence presentation and to guide the group as far as possible towards consensus (Street et al. 2014). These factors are linked to the principles of quality deliberation (Table 13.1), particularly information, substantive balance and diversity.

Deliberative democratic theory (which underpins these methods) suggests that, in order to have sound and undistorted consideration of the issues, participants in the process need to come to the forum with an open mind, that is, they are ready and willing to engage with the evidence and diversity of experience and, if necessary, to shift their own views (Bohman and Rehg 1997; Dryzek 2000; Fishkin 2009). This relates to two other factors of quality deliberation: conscientiousness and equal consideration.

Therefore, vested and 'partisan' stakeholders are usually excluded from deliberative forums since there is a danger that they may skew the debate. A partisan or vested stakeholder is someone with strongly held beliefs on a topic who is unable or unwilling to shift in response to compelling evidence or alternate views and/or has a vested interest in the outcome of the decision-making (Chap. 3). This includes those individuals:

1. Representing or identifying strongly with a group which holds strong views, e.g. patient advocate, clinician advocate or health technology developer lobbyist
2. With vested financial interest, e.g. industry lobbyist and in some cases clinicians
3. With strongly held views due to personal experience or because the decision will have a major impact on their lives, e.g. caregivers and patients

An individual in the first group may experience a shift in personal opinion as a result of their exposure to the deliberation, but may not be able to shift the perspective they display publicly because of their sense of loyalty or duty to the stakeholders they represent. Individuals in the second group may be less or more willing to shift based on their financial stake in the debate. Individuals in the third group,

notably caregivers and patients, may be more flexible in their willingness to shift their attitudes depending on the nature of the impact; for example, some technologies may be life-saving.

13.2.2 Principles of Democracy

Fishkin (2009) defines democracy as requiring four basic principles: political equality, deliberation, mass participation and non-tyranny (of the majority or the minority). Different models of public participation in democratic systems require trade-offs between these four principles. According to Fishkin (2009), there are four distinct ways in which democracy can be delivered (some models are more akin to mass democracy whereas others are closer to deliberative democracy):

- *Competitive democracy* (through competitive elections)—in this model, individual preferences count, but there is no place for mass public participation in public decision-making. This is, in part, because there is a fear that participation from the mass public, with raw (variably informed) opinions, could result in tyranny. This model reflects the current democratic system of representative electoral voting.
- *Elite deliberation*—where decisions are filtered through an elite or expert group which is not usually representative of the broader population. In this model, quality deliberation and non-tyranny are valued, but little value is placed on political equality or mass participation.
- *Participatory democracy*—where direct consultation with the public (and hence political equality and mass participation) is valued, but there may be less opportunity for deliberation, and therefore it could result in tyranny.
- *Deliberative democracy*—which focuses on a combination of political equality and deliberation. The typical public involvement process for this model is microcosm deliberation, also called a mini-public, in which a descriptively representative sample of the population can take part. As such, although tyranny is unlikely, there may be limited opportunity for mass participation.

13.2.3 Patient Involvement in Deliberative Methods in HTA

Based on Fishkin's work, it is possible to chart the different models of public participation, including patients, in HTA as shown in Fig. 13.1.

In HTA, representation of patient views through patient representatives would fall under the elite deliberation end of the spectrum (i.e. a 'select group') with or without exposure to the views of people outside the patient group. In the first scenario (top right of the figure), patient representatives sit on panels of experts which deliberate on information provided through systematic reviews of evidence

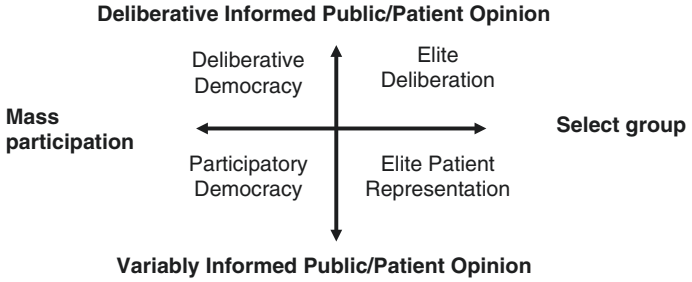

Fig. 13.1 Patient/public participation in HTA

relevant to a technology. Such patient representatives are usually broadly representative of 'patients'. They may bring their own personal experience to the discussion or may act to synthesise and channel the voices of patient groups associated with the particular technology under discussion (Lilly 2009). In the second scenario (bottom right of the figure), patient representatives from patient organisations advocate on behalf of particular groups of patients (Rabeharisoa et al. 2013). Participatory democracy (bottom left of the figure) potentially involves patients from across the patient spectrum including patients who sit outside organised patient groups and those using public health technologies, such as screening tests or vaccines. In some cases, although systems may be potentially broad in their involvement, poorly funded and designed systems may preclude non-aligned patients from being involved (Lopes et al. 2016). Deliberative inclusive methods emerge from the democratic pathway described in the top left quadrant, albeit with a small group selected to represent the broad mix of population views rather than community-wide deliberation (although this may follow on from the dissemination of forum recommendations). Deliberative forums or mini-publics with a small group offer an informed inclusive way of incorporating patient voices into decision-making in HTA. Mini-publics include citizen juries, consensus conferences and citizen councils. These are usually small diverse groups of 12–40 people brought together to consider a contentious issue, for example, whether public funds should be allocated to a technology or, more broadly, what values should underpin funding such decisions.

Mini-publics have been rarely used directly in HTA, and where this has occurred, the focus has been on the participation of citizens rather than patients (Menon and Stafinski 2008; Barham 2012; Stafinski et al. 2014a, b). However, Paul et al. (2008) recruited *prospective* patients in their study which examined public opinion on the use of breast mammography for detection of breast cancer in women aged 40–50. In addition, mini-publics have been used to inform health policy in other areas where the public may be seen as prospective patients or existing patients as well as non-aligned citizens, for example, in the distribution of vaccine in a pandemic (Braunack-Mayer et al. 2010), in the development of a biobank (Burgess et al. 2008) or in the funding of new technologies (Dunkerley and Glasner 1998; Chafe et al. 2010).

13.2.4 Including Patient Views and Experience in Deliberative Processes

In some jurisdictions (e.g. Public Partners in the Scottish Medicines Consortium), it is common for patient representatives to present patient views and experience for consideration in an elite deliberative HTA forum of clinical, economic and policy experts. However, there is usually little opportunity for patients and patient organisations to engage in their own deliberation before presenting this information. If such deliberation were supported, it might permit better identification of priorities across a patient group.

Patient views may also be considered in the evidence presentation for deliberative methods involving non-aligned citizens (mini-publics). For example, representative patients or patient advocates may be asked to present their views and experience to a mini-public as part of the evidence needed for citizens to make a balanced decision even while they may be excluded from sitting on the forum itself (Stafinski et al. 2014a, b). This bears examination within the context of deliberative democratic theory and application of deliberative methods to patient involvement in HTA. For example, equal consideration is a key element in deliberative democratic theory, and confining the involvement of patients to the elicitation of patients' perspectives for presentation to a deliberative forum of citizens may compromise this focus. In addition, the views of one or a very small number of 'representative' patients may not adequately represent the patient experience at large.

An alternative to this is the use of parallel deliberative forums with a range of stakeholders including patients. This was the basis for the ASTUTE project, a programme of multi-stakeholder engagement around questions of public funding for health technologies deemed to deliver little or no health gain for their cost (Watt et al. 2012). Within the ASTUTE project, Hodgetts et al. (2014) reported the use of multiple deliberative engagements with groups of patients, clinicians and community members in the case study of public funding for assisted reproductive technologies (ART). The findings from the different groups fed into each other in a second round of deliberation with all the findings funnelled through a final round of a citizens' jury. One of the complexities encountered was the difficulty in recruiting patients who had been 'unsuccessful' in their ART journey in part because it might be considered unethical to do so but also because these patients were reluctant to engage. Difficulties in recruiting appropriate patients are present in all areas of patient involvement in HTA but may be most marked in those methods which require a longer time commitment or where the engagement is physically or mentally taxing, even where involvement is financially supported.

Herbison et al. (2009) took a more focused approach. In their study, a 'citizens' jury' model was used to explore patient views in developing research priorities in urinary incontinence. The women were not necessarily 'patients' since only half of the group had sought treatment but were rather 'women living in the community with urinary incontinence'. As with the ART study reported above, Herbison et al.

(2009) also described the difficulty in recruiting a diverse group of patients with a range of experience with a particular health condition.

13.3 Ethical Issues in Involving Patients in Deliberative Methods

Involving patients in HTA may be challenging. In particular, it may be difficult to capture the experience and views of patients with rapidly progressing conditions, with conditions in which the patient's ability to communicate is challenged or with conditions where patients need constant ongoing support or where they are often unwell. In the case of deliberative inclusive methods, participants may be required to attend for 1–2 days in order to allow effective informed deliberation with others. This may not be possible for some patients and caregivers.

Similarly, face-to-face discussion of life-changing or life-saving technologies may be particularly taxing for many patients. In the case of individuals recruited for the ASTUTE study, patients currently undergoing or intending to undergo assisted reproductive technologies were excluded from recruitment as were patients who had undergone such procedures less than 2 years before (Hodgetts et al. 2014). This approach was intended to ameliorate the potential distress to participants.

13.4 Strengths and Limitations of the Method in HTA

Deliberative inclusive methods are usually considered time-consuming and expensive although truncated juries as described in Street et al. (2014) may be of comparable cost to alternative quantitative or qualitative methods for inclusion of patient views and perspectives in HTA. The strength of deliberative inclusive methods lies in their close alignment with the policy process, in the ability to involve a diverse group patients (and/or citizens) in an informed process which reaches for a consensus policy outcome and in the capacity building and empowerment for citizens inherent in the process. In an era when trust in government is eroding (Dalton 2005), the use of deliberative methods to involve patients in policy development may go some way to rebuilding community confidence.

However, government and commercial confidentiality which characterises the HTA process may impede conduct of a fully informed deliberative process. Frequently, there is considerable information which is not in the public domain, and potentially none or only some of that information will be made available to participants in a deliberative forum. In this circumstance, the consequence is that participants will not have the full detailed picture for their decision-making, and this information may be considered crucial by governments in making the decision. Clearly this would limit the credibility of the deliberative process in the eyes of policymakers.

Deliberative democratic theory emphasises the importance of clear pathways between the forum recommendations and influence in the policy sphere. Even with direct engagement with governments or decision-making bodies, influence may be lacking. Government involvement in any activity that includes a community involvement component is inherently risky. Certainly a government may not wish to be linked to findings that it does not wish to be seen to sanction. Finally, because of the small number of community members usually involved in these methods, many policy advisors and politicians may display ambivalence about the involvement of community in decision-making through deliberative forums.

13.5 Conclusions

Deliberation in which participants have access to accurate and substantive information relevant to the discussion and conscientiously consider the merits of the arguments and evidence presented is an essential part of the HTA process. What may be lacking in some jurisdictions, where patient and citizen involvement is tokenistic, is the diversity of views reflecting the range of patient experience. Deliberative inclusive methods which include patients and caregivers deliver that diversity. Perhaps even more importantly they offer a potential resolution for contentious value-laden decisions in HTA. In particular, they can provide the rationale for patient- and/or public-informed recommendations which may differ to those provided by elite deliberation. To date, the methods have been rarely used to fully involve patient and caregivers in the HTA process. Areas in which their use might be most beneficial include public funding for emerging high-cost cancer medicines, prostate and bowel cancer screening programmes and the delivery of ‘smart’ technologies which allow the elderly to remain at home for as long as possible. For quality deliberation to occur in HTA, substantive inclusion of the perspectives of patients and caregivers is essential. Deliberative inclusive methods which include patient/caregiver forums potentially offer the ‘gold standard’ in this respect.

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References

Barham L. Public and patient involvement at the UK National Institute for health and clinical excellence. Patient. 2012;4:1–10.

Bohman J, Rehg W. Deliberative democracy: essays on reason and politics. Cambridge, MA: MIT Press; 1997.

Braunack-Mayer AJ, Street J, Rogers W, Givney R, Moss J, Hiller J. Including the public in pandemic planning: a deliberative approach. BMC Pub Health. 2010;10:1–9.

Burgess M, O'Doherty K, Secko D. Biobanking in British Columbia: discussions of the future of personalized medicine through deliberative public engagement. Personalized Med. 2008;5:285–96.
Chafe R, Merali F, Laupacis A, Levinson W, Martin D. Does the public think it is reasonable to wait for more evidence before funding innovative health technologies? The case of PET scanning in Ontario. Int J Technol Assess Health Care. 2010;26:192–7.
Dalton RJ. The social transformation of trust in government. Int Rev Soc. 2005;15(1):133–54.
Dryzek J. Deliberative democracy and beyond: liberals, critics, contestations. New York: Oxford University Press; 2000.
Dunkerley D, Glasner P. Empowering the public? Citizens' juries and the new genetic technologies. Crit Pub Health. 1998;8:181–92.
Fishkin J. When the people speak. Oxford: Oxford University Press; 2009.
Goodin RE, Dryzek JS. Deliberative impacts: the macro-political uptake of mini-publics. Polit Soc. 2006;34:219–44.
Hendriks C. Deliberative governance in the context of power. Polit Soc. 2009;28:173–84.
Herbison P, Hay-Smith J, Paterson H, Ellis G, Wilson D. Research priorities in urinary incontinence: results from citizens' juries. BJOG. 2009;116:713–8.
Hodgetts K, Hiller JE, Street JM, Carter D, Braunack-Mayer AJ, Watt AM, et al. Disinvestment policy and the public funding of assisted reproductive technologies: outcomes of deliberative engagements with three key stakeholder groups. BMC Health Serv Res. 2014;14:204–17.
Lilly E. Enhancing Consumer Involvement in Medicines Health Technology Assessment (Final Report), Deloitte Touche Tohmatsu Ltd; 2009. p. 89.
Lopes E, Street J, Carter D, Merlin T. Involving patients in health technology funding decisions: stakeholder perspectives on processes used in Australia. Health Expect. 2016;19:331–44.
Mansbridge J, Bohman J, Chambers S, Estlund D, Føllesdal A, Fung A, et al. The place of self-interest and the role of power in deliberative democracy. J Polit Philos. 2010;18:e100.
Menon D, Stafinski T. Engaging the public in priority-setting for health technology assessment: findings from a citizens' jury. Health Expect. 2008;11:282–93.
Ozanne JL, Corns C, Saatcioglu B. The philosophy and methods of deliberative democracy: implications for public policy and marketing. J Pub Policy Market. 2009;28:29–40.
Paul C, Nicholls R, Priest P, McGee R. Making policy decisions about population screening for breast cancer: the role of citizens' deliberation. Health Policy. 2008;85:314–20.
Rabeharisoa V, Moreira T, Akrich M. Evidence-based activism: patients' organizations, users' and activist's groups in knowledge society. Centre Sociol Innovat. 2013;33:1–27.
Stafinski T, McCabe C, Menon D. Determining social values for resource allocation decision-making in cancer care: a Canadian experiment. J Cancer Policy. 2014a;2:81–8.
Stafinski T, Menon D, Yasui Y. Assessing the impact of deliberative processes on the views of participants: is it 'in one ear and out the other'? Health Expect. 2014b;17:278–90.
Street J, Duszynski K, Krawczyk S, Braunack-Mayer A. The use of citizens' juries in health policy decision-making: a systematic review. Soc Sci Med. 2014;109:1–9.
Watt AM, Hiller JE, Braunack-Mayer AJ, Moss JR, Buchan H, Wale J, et al. The ASTUTE health study protocol: deliberative stakeholder engagements to inform implementation approaches to healthcare disinvestment. Implement Sci. 2012;7:101–12.

Chapter 14
Analysis of Social Media

Jackie Street and Lucy Farrell

14.1 Introduction

The Internet has become a widely used resource for patients living with a range of health conditions. Social media technologies enable patients to interact with others with similar conditions for the purposes of sharing information, advice and support irrespective of time or location (Colineau and Paris 2010; Walther and Boyd 2002). Online communities are used by diverse patient groups including those with chronic diseases such as cancer and diabetes, mental health disorders, rare conditions, and stigmatised illnesses, as well as carers (informal caregivers) of these patients. Analyses of patient interactions on social media offer new (and as yet largely unrealised) opportunities for patient involvement in HTA, including to collect population perspectives on broad health-related issues and to connect with patient experiences. This chapter explores the use of social media analysis for providing patients' perspectives and experiences in the HTA context. It discusses the possible applications of social media analysis to inform HTA and reflects on methodological and ethical considerations for researchers.

14.2 Use of Social Media to Elicit Patients' Perspectives

Social media are web-based technologies which enable individuals and communities to generate, share and comment on content (Kietzmann et al. 2011). These include social networking sites such as Facebook, LinkedIn, Twitter and Instagram, as well as blogs, and discussion forums on websites or mobile phone apps. These technologies have enabled the development of online patient

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communities where individuals can come together to give and receive information and support. Studies of online patient communities demonstrate that these interactions offer substantial benefit to patients in the form of emotional support, access to health information, and help with treatment decisions (Moorhead et al. 2013; Wicks et al. 2010).

The use of social media to engage with citizen perspectives is becoming increasingly common in the public sector (Magro 2012) as well as to gather the views of the general public on health issues and interventions (Farrell et al. 2015; Giles et al. 2015; Street et al. 2011). However, the analysis of existing social media to engage with patient views or the use of social media to involve patients directly in the development of health policy and practice has been more limited. Social media analysis presents an opportunity for HTA researchers to gain insight into patients' treatment decisions and the real-time impact of health conditions on quality of life. Additionally, it may increase understanding of patients' experiences with particular technologies and provide patient views on how to improve service provision. Online methods offer particular value in enabling engagement with patient groups that may not be possible in other research settings such as interviews or focus groups. This is especially the case for the housebound, rare conditions or where geographical limitations may impede the formation of more traditional patient organisations, as well as for sensitive health issues.

A limited body of work has used social media analysis to deliver information about patients' perspectives and experiences for the purposes of HTA (Merlin et al. 2011; Street et al. 2008; Street et al. 2011). Since time is often short in the HTA cycle, obtaining ethics approval, recruiting participants and conducting a rigorous qualitative study with patients can be difficult. Social media offers opportunities to conduct relatively fast, inexpensive and feasible qualitative research for gauging patient experience with an existing technology or service (Merlin et al. 2011). It may be less useful for explicating patient views about the value of new technologies but may provide insight into the shared needs and values of a particular patient community (Street et al. 2008). Involving patients in HTA through a social media platform is an inexpensive way of gauging patient views and experience and is particularly useful for 'hard-to-reach' groups such as the housebound. It may also identify issues which can then be explored in more depth through focus groups and interviews with patients.

However, perhaps the real value of social media is that it can be an especially good way of engaging with the particular expertise held by patients. As Hartzler and Pratt (2011) indicate, a valuable aspect of patient expertise is the detailed understanding of the lived experience of a disease. Social media offers a platform in which the conversation about priorities and experience around a health technology can be led by patients rather than clinicians and researchers. Such conversations can 'facilitate a natural expression of patient expertise and provide contextual detail' (Hartzler and Pratt 2011, p. 13) which may be invaluable for HTA.

14.3 Methods of Collection and Analysis

The ongoing development of social media platforms and constantly changing cultural contexts of the Internet use defy attempts to comprehensively document research methods for eliciting patients' perspectives via social media. Instead, we distinguish two broad methodological categories (*observational* and *participatory*), each encompassing a range of possible approaches. Some methodological considerations for each of these approaches are presented.

14.3.1 Observational Methods

14.3.1.1 Qualitative Approaches

Naturally occurring social interactions on the Internet provide a rich source of data for researchers collecting patient views and experience to inform HTA. Qualitative observational research methods for studying the interactions and views of online communities have been termed 'netnography' (Kozinets 2010), in reference to the adaption of ethnographic research techniques to study web-based communities. Kozinets (2010) describes the 'complex dance' between Internet technology and culture which is driving major social change and which can only be fully understood through ethnography (Chap. 12). Researchers employing qualitative observational netnographic methods may do so covertly, by 'lurking' on social media platforms to observe and record interactions. These methods depart from more traditional research methods for eliciting patient views such as focus groups or interviews as the data obtained is not provided to the researcher and the research participants may not intend, or even be aware, that the data they created is being used for research purposes. Compared with other methods for eliciting patient views, observational online methods are uniquely unobtrusive, owing to the lack of interaction between researcher and participant. These features also make netnography a rapid and inexpensive method for collecting data to inform HTA or at least to reveal particular issues and questions which might then be explored further through direct interaction with patient organisations.

The following procedures are suggested for qualitative observational netnographic research (based on Kozinets 2010 and Elliott et al. 2005):

1. Selection of appropriate social media forum(s): this should be guided by (1) the relevance of the forum to the research questions, (2) high 'traffic' of postings, (3) many discrete participants, (4) detailed or descriptively rich data and (5) interactivity (if required by the research question).
2. Data collection: as online data are often plentiful and easy to obtain, judicious management of surplus data is an important consideration. Inclusion and

exclusion criteria and data management procedures should be established in advance of data download.

3. Analysis and interpretation: coding and contextualisation of data. Traditional qualitative textual analysis methods such as thematic and discourse analysis are transferable to data collected online (Hooley et al. 2012). However, as textual interactions lack some social nuances, traditional analytic methods may need to be adapted (Stewart and Williams 2005). Qualitative research software such as QSR NVivo can expedite coding and analysis.

Providing a definitive method for searching for online blogs, discussion forums, tweets or communities is difficult since the online environment is fluid and changes rapidly. Some suggestions for locating patient interactions on social media are:

- Google searching using relevant keywords associated with a disease or condition in association with a domain name, e.g. blogspot.com
- Searching with keywords inside relevant discussion forums associated with advocacy or patient organisations
- Google searching using key terms describing types of social media (e.g. discussion forum) in association with relevant keywords describing a disease condition
- Searching within websites dedicated to patients with user-generated comment, e.g. PatientsLikeMe (https://www.patientslikeme.com/), or within social media platforms (e.g. Facebook).

Observational social media analysis to elicit patient views can provide access to a diverse range of opinions and concerns. The methods can be particularly useful in HTA for gathering patient views about sensitive health topics that may be rarely spoken about in face-to-face settings, where patients may be difficult to recruit for research purposes (Elliott et al. 2005). Additionally, these methods may be useful where social desirability – that is, the expression of views at odds with the niceties of social acceptability – might impede the diversity of views expressed (Farrell et al. 2015). The method allows the collection of real-time rich data of patient experience over a long period rather than relying on recall. Some blogs record a patient's experience over many years, for example, blogger Kerri Sparling has written regularly about her experience of living with type 1 diabetes from the age of 26 (2005) to the time of this publication (Sparling 2016).

However, there are issues with qualitative analysis of social media data which need consideration: currently active social media users are concentrated in developed countries, in urban areas and in younger age groups (Poushter and Stewart 2016), and high prevalence diseases are more likely to be better represented. The applicability of the experience of patients in other countries to the experience in the country commissioning the HTA may be questioned, although evidence from published journal articles often draws on such experience. To a degree, it can be argued that the patient experience is a universal one.

Discussion forums attached to online media articles may be a useful source of patient views for HTA particularly in disinvestment policy scenarios when a government wishes to reduce or remove funding from an established technology or service

(Street et al. 2011). However, owing to the anonymity of the online environment, it may not be possible to verify the authenticity of the patient voices collected or whether they are impartial from the influence of the health technology developer or clinician groups. Despite the shortcomings, qualitative social media analysis can be useful as a triangulation point with views gleaned from other sources such as peer-reviewed journal articles, news media sources, surveys or face-to-face qualitative research. Triangulation, which refers to the use of multiple sources, methods, researchers and theories in a research project, is used to 'overcome the intrinsic bias from single-method, single-observer and single-theory studies' (Denzin 1989, p. 307) and, importantly for HTA, to assist the researcher in understanding complex areas of policy and practice (Liamputtong and Ezzy 2005, p. 40). For example, in attempting to gauge patient views, it is probable that people who engage with some forms of social media, for example, extreme views expressed in discussion forums, may not engage with traditional research approaches. Differences between the sources may indicate potential areas for future research.

14.3.1.2 Quantitative Approaches

Some social media sources such as Twitter, Facebook and Instagram encourage frequent, short postings which may be copious and individually offer little informational value for researchers concerned with collecting patient views. However, the aggregation of many posts can generate important knowledge by providing a snapshot of views about topics of interest.

Large quantities of data can be collected from social networking sites via keyword and phrase searching (for instance, via the Twitter Application Programming Interface: https://dev.twitter.com/rest/public). Studies have employed quantitative analysis of data from social networking sites to measure political sentiment (Barbosa and Feng 2010) and to gather information and gauge public concerns about health issues such as the H1N1 influenza pandemic (Chew and Eysenbach 2010) and community understandings of wellness (Martz 2015). Analysis of social network data can be performed manually or can be automated (Pak and Paroubek 2010). This offers potential for HTA researchers to monitor sentiment about a given issue in near real time as well as allow changes in opinions over time to be identified via longitudinal analysis. These methods offer significant time and cost advantages over tracking changes in opinions through more traditional opinion research methods, such as surveys.

14.3.2 Participatory Methods

14.3.2.1 Qualitative Approaches

Online qualitative participatory methods, such as interviews and focus groups, are online discussions which involve interactions between researcher(s) and participant(s) in order to explore views, experiences and motivations around a particular topic.

These methods involve the translation of traditional qualitative research methods to the online environment (Hooley et al. 2012). As with traditional methods, these discussions can be structured, semi-structured or unstructured to enable participants' greater or lesser control over the content (Gaiser 2008; Tates et al. 2009).

When conducted online, these discussions can occur synchronously (i.e. participants and researchers contribute to the focus group or interview in real time) or asynchronously (participants and researchers can engage in the discussion at times of their own choosing; Hooley et al. 2012; Stewart and Williams 2005). Synchronous discussions typically use tools such as chat rooms, messenger services or video applications such as Skype, which enable participants and researchers to interact in real time. Asynchronous discussions tend to use discussion boards or forums, email and social networking sites. There is a scope for overlap with these technologies: 'Twitter chats', for instance, are synchronous moderated discussions which bring participants together at a scheduled time to discuss a particular topic using a hashtag especially designated for the event.

A particular form of the use of social media for patients' perspectives which uses participatory approaches is the compilation of prototypical patient stories. This approach has been used by the Canadian Organisation of Rare Disorders (CORD). CORD engaged with patients through its Facebook page to collect experiences with new treatments and invited patients to submit their stories. These were then used to compile a prototypical patient story which was posted online for validation and comment (Wong-Rieger 2010). Social media may also be used to share surveys and co-produced documents such as draft-completed submission templates for submission to assessment processes.

Online participatory qualitative research methods offer advantages over traditional face-to-face approaches in terms of cost, time and flexibility for both participants and researchers. As well, some of the imbalances of power that may occur in qualitative research settings between researchers and participants can be reduced by conducting research online (Seymour 2001). Online methods can therefore engender more egalitarian and participatory research than is possible through face-to-face methods. These factors mean that online participatory methods may be particularly valuable for eliciting patient views, especially where health conditions may pose challenges for face-to-face communication. In particular, asynchronous methods offer patients the ability to manage the timing and location of their participation in research projects. However, these methods also pose challenges for qualitative researchers: recruitment of participants is limited by the demographics of social media users, and building rapport with participants can be difficult in online environments.

14.3.2.2 Quantitative Approaches

Online surveys are perhaps the most obvious methods to collect patient views to inform HTA. Surveys can be developed within social networking sites (e.g. using Facebook or Twitter survey apps), using online survey software, or on blogs or websites.

The basic principles of survey design, including question development and sampling, are consistent regardless of whether the survey is conducted online or in another format. Online surveys enable views to be collected from large and diffuse patient populations and offer a number of benefits over surveys conducted face to face, on paper or via telephone:

- Data collection can occur quickly over a large geographical area.
- Online surveys are low cost, as interviewers are not required.
- Questionnaire design is flexible. For example, images, personalised questions and complex question sequencing can be included.
- Easy for participants to use, as timing and location of participation can be managed by participants themselves.

However, online surveys have some significant limitations including issues with sampling and non-response bias (Evans and Mathur 2005). Despite rigorous recruitment efforts, response rates may be very low (Mitchell et al. 2014). These issues generally mean that online surveys cannot be considered representative of the views of a given population, which is often a key objective for survey research. As such, careful consideration should be given to the appropriateness of online surveys for addressing specific research questions.

14.4 Ethical Considerations of Social Media Analysis to Collect Patients' Perspectives

Social media research presents unique ethical considerations. What is private and what is public is central to ethical research practice; however, these lines can be blurred for social interactions occurring on the Internet. This has implications for the important research ethics issues of informed consent and participant privacy.

For participatory research using social media, informed consent and participant privacy can be managed through the translation of traditional research ethics protocols to the online environment. This may involve emailing study information and consent forms to participants, prefacing surveys with a mandatory consent question or posting consent forms and providing links to further information about the study on Facebook pages, websites, blogs, etc. Participant privacy can be protected through the use of pseudonyms self-selected by participants prior to participation in the research or subsequently allocated by the researcher. Consideration may also need to be given to ownership of data produced online, how these will be archived and whether unauthorised access may be possible in the short or long term.

Ethical considerations are more complex for observational online methods. Whether online interactions can ethically be used as research data without informed consent hinges on whether social media interactions are considered public or private. If publicly available, some researchers argue that social media interactions are a legitimate source of research data akin to other forms of public media. Others draw attention to individuals' expectations of privacy in their participation in online

communities and the distress that can be imposed by research 'intruders' (Eysenbach and Till 2001).

Kozinets (2010) advocates a cautious approach to the private-versus-public issue, including full disclosure of the researcher's presence and intentions, obtaining informed consent and ensuring confidentiality. This may occur prospectively by requesting permission to observe online interactions and giving community members the opportunity to withdraw from the social media forum for the period of the study or, retrospectively, by contacting individuals in order to obtain their consent to replicate postings.

However, informed consent may be considered impractical or overly stringent for discussions taking place in publicly accessible forums. Disclosing the research project in advance of data collection may influence community interactions, while seeking consent retrospectively may not be possible where posts are made anonymously or where contact details are not available. Further, informed consent is likely to be impossible to obtain for quantitative observational data collection where large volumes of data are analysed.

Covert observation of social media interactions may be appropriate in some circumstances, particularly where patient views about sensitive or rare health conditions cannot be obtained through other methods. Where informed consent is not possible, particular care should be taken to protect participant privacy through the use of pseudonyms, composite quotes and suppression of the characteristics of the social media site. Even with these measures, it may be possible to locate participant details by tracing quotes in Google.

Considerations in social media research ethics should therefore be considered in terms of the specific research context. Eysenbach and Till (2001) propose the following considerations to guide ethical online research practice:

- Intrusiveness: is the researcher a passive observer or an active participant in the online community being researched?
- Perceived privacy: what are the community's expectations of privacy?
- Vulnerability: how vulnerable is the community (e.g. support forums for victims of sexual abuse or AIDS patients will be highly vulnerable)?
- Potential harm: as a result of the above, is the use of data for research purposes likely to harm individuals or the online community?

Finally, the researcher must also consider the potential for backlash from the online community and possible online harassment in retaliation for intrusion into the community's domain. Researchers may need to consider taking particular care in which details they reveal about themselves in any online interactions.

14.5 Strengths and Limitations of Social Media Analysis for HTA

Social media analysis can provide rich insights to inform HTA, by providing access to a diverse range of opinions and concerns and by providing a depth and immediacy of data which may be impossible in other research contexts. Online methods

enable the collection of views which may be prevalent in the community but which may not be able to be collected through traditional research methods due to participants' moderation of the views they express in their interactions with others. This is particularly pertinent to analyses of patients' perspectives on embarrassing or stigmatised health conditions, where the perception of anonymity and detachment from social cues and consequences that can occur online (Suler 2004) may engender more open and frank discussion of patient concerns. However, this 'online disinhibition effect' may also present some challenges for social media analysis, as higher levels of hostility or aggression may be expressed than would occur in a face-to-face research setting. Careful moderation of the research process is therefore required in order to reduce harm to participants.

Social media analysis and the use of social media platforms enable the involvement of hard-to-reach populations in HTA processes. For example, patients with rare diseases are often scattered geographically. Social media provides these patients with a channel for their 'voice' and to 'gather' virtually.

The anonymity of the online environment can pose unique challenges for researchers examining patient experiences. As the identity and personal characteristics of social media users cannot be ascertained, it is possible for medically well individuals to fake illness online (Pulman and Taylor 2012), or patients may deliberately or inadvertently adopt multiple identities in patient forums (Ann Single, personal communication). It is therefore important for researchers analysing social media to elicit patients' perspectives to consider the legitimacy of the views gathered. The use of online forums attached to recognised patient organisations may increase the legitimacy of the data collection in the eyes of HTA stakeholders.

The representativeness of views obtained from research conducted online may also pose problems for researchers. The 'digital divide' may impede the collection of views from people on low incomes, who are unemployed, living in remote areas, with low literacy levels, of Indigenous heritage, or who are older, as these groups are most likely to lack access to the Internet (Australian Bureau of Statistics 2016). As ill health is socially patterned, the use of online methods to elicit patients' perspectives may preclude the views of important patient groups.

14.6 Conclusion

Particularly in the case of 'hard-to-reach' patient groups such as those diagnosed with rare diseases and embarrassing or stigmatised conditions, social media analysis provides an invaluable opportunity to tap into patient experiences and perspectives which may be extremely difficult to collect outside of this space. It is also a rapid and inexpensive way of collecting rich in-depth data for more common conditions such as diabetes, cancer and infertility. While there are important ethical and methodological challenges involved with collecting patient views and experience via social media, the increasing penetration of these technologies into everyday life will increase the potential for using these tools to inform HTA.

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References

Australian Bureau of Statistics. Household Use of Information Technology, Australia, 2014-15. Cat. No. 8146.0. Canberra: Commonwealth of Australia; 2016.

Barbosa L, Feng J. Robust sentiment detection on twitter from biased and noisy data. In: Proceedings of the 23rd International Conference on Computational Linguistics: Posters. Association for Computational Linguistics; 2010. p. 36–44.

Chew C, Eysenbach G. Pandemics in the age of twitter: content analysis of tweets during the 2009 H1N1 outbreak. PLoS One. 2010;5:e14118.

Colineau N, Paris C. Talking about your health to strangers: understanding the use of online social networks by patients. New Rev Hypermedia Multimed. 2010;16:141–60.

Denzin NK. The research act: a theoretical introduction to sociological methods. 3rd ed. Englewood Cliffs, NJ: Prentice Hall; 1989.

Elliott R, Shankar A, Langer R, Beckman SC. Sensitive research topics: netnography revisited. Qual Mark Res. 2005;8:189–203.

Evans JR, Mathur A. The value of online surveys. Int Res. 2005;15:195–219.

Eysenbach G, Till JE. Ethical issues in qualitative research on internet communities. BMJ. 2001;323:1103–5.

Farrell LC, Warin MJ, Moore VM, Street JM. Emotion in obesity discourse: understanding public attitudes towards regulations for obesity prevention. Sociol Health Illn. 2015;38:543–58.

Gaiser TJ. Online focus groups. In: Fielding NG, Lee RM, Blank G, editors. The sage handbook of online research methods. London: Sage; 2008. p. 290–306.

Giles E, Holmes M, McColl E, Sniehotta FF, Adams JM. Acceptability of financial incentives for breastfeeding: thematic analysis of readers' comments to UK online news reports. BMC Pregnancy Childbirth. 2015;15:1.

Hartzler A, Pratt W. Managing the personal side of health: how patient expertise differs from the expertise of clinicians. J Med Internet Res. 2011;13:e62.

Hooley T, Wellens J, Marriott J. What is online research? Using the internet for social science research. New York: Bloomsbury; 2012.

Kietzmann JH, Hermkens K, IP MC, Silvestre BS. Social media? Get serious! Understanding the functional building blocks of social media. Bus Horiz. 2011;4:241–51.

Kozinets RV. Netnography: doing ethnographic research online. London: Sage Publications; 2010.

Liamputtong P, Ezzy D. Qualitative research methods. South Melbourne: Oxford University Press; 2005.

Magro MJ. A review of social media use in e-government. Adm Sci. 2012;2:148–61.

Martz P. An observational study of social media technology conversations: exploring how members of the Alberta public, organizations and health care professionals express wellness, in relation to children. Dissertation. University of Alberta. 2015.

Merlin T, Street J, Holton C, Juneja V, Tamblyn D, Ellery B, Scrimgeour S, Hennessy S. Review of MBS items for specific ophthalmology services under the MBS quality framework for consideration by medical services advisory committee. Canberra, ACT: Commonwealth of Australia; 2011.

Mitchell J, Callaghan P, Street J, Neuhaus S, Bessen T. The experience of melanoma follow-up care: an online survey of patients in Australia. J Skin Cancer. 2014;2014:429149.

Moorhead SA, Hazlett DE, Harrison L, Carroll JK, Irwin A, Hoving C. A new dimension of health care: systematic review of the uses, benefits, and limitations of social media for health communication. J Med Internet Res. 2013;15:4.

Pak A, Paroubek P. Twitter as a corpus for sentiment analysis and opinion mining. In: Proceedings of the seventh conference on international language resources and evaluation (LREC'10, May 2010); 2010. p. 1320-1326.

Poushter J, Stewart R. Smartphone ownership and internet usage continues to climb in emerging economies but advanced economies still have higher rates of technology use. Pew Research Center. February 2016. http://www.pewglobal.org/2016/02/22/smartphone-ownership-and-internet-usage-continues-to-climb-in-emerging-economies/. Accessed 11 Oct 2016.

Pulman A, Taylor J. Munchausen by internet: current research and future directions. J Med Internet Res. 2012;14:4.

Seymour WS. In the flesh or online? Exploring qualitative research methodologies. Qual Res. 2001;1(2):147–68.

Sparling KM. Six until me. 2016. http://sixuntilme.com/wp/. Accessed 28th July 2016

Stewart K, Williams M. Researching online populations: the use of online focus groups for social research. Qual Res. 2005;5:395–416.

Street JM, Braunack-Mayer AJ, Facey K, Ashcroft RE, Hiller JE. Virtual community consultation? Using the literature and weblogs to link community perspectives and health technology assessment. Health Expect. 2008;11:189–200.

Street JM, Hennessy SE, Watt AM, Hiller JE, Elshaug AG. News and social media: windows into community perspectives on disinvestment. IJTAHC. 2011;7:376–83.

Suler J. The online disinhibition effect. Cyberpsychol Behav. 2004;7:321–6.

Tates K, Zwaanswijk M, Otten R, van Dulmen S, et al. Online focus groups as a tool to collect data in hard-to-include populations: examples from paediatric oncology. BMC Med Res Methodol. 2009;9:1.

Walther JB, Boyd S. Attraction to computer-mediated social support. In: Lin CA, Atkins D, editors. Communication technology and society: audience adoption and uses. Cresskill, NJ: Hampton Press; 2002. p. 153–88.

Wicks P, Massagli M, Frost J, Brownstein C, Okun S, Vaughan T, et al. Sharing health data for better outcomes on PatientsLikeMe. J Med Int Res. 2010;12:e19.

Wong-Rieger, D. HTAi 7th Annual Meeting, Dublin. 2010. Panel Session 19.

Chapter 15
Qualitative Evidence Synthesis

Andrew Booth

15.1 Introduction

Qualitative evidence synthesis, also known as qualitative systematic review, offers a vehicle for presenting patients' attitudes, beliefs and feelings as originally captured by individual qualitative research studies. By aggregating or integrating views from multiple studies, rather than a single study, the science of systematic reviews takes steps to protect against allowing findings from an isolated study to overly influence our understanding or even to lead us to omit important perspectives. This chapter examines the wide range of uses to which qualitative evidence synthesis can be applied within HTA (Ring et al. 2011a, b) and introduces methods to identify, synthesise and analyse patient narratives from the research literature. The chapter concludes by briefly reviewing methods by which qualitative data might be integrated with quantitative data from an effectiveness review.

The power of a single patient's voice is not to be underestimated. However, as each individual patient represents a composite of unique experiences, attitudes, opinions and values, there is even greater power to be harnessed from numerous patient accounts collected and interpreted through accepted methods of qualitative data collection and analysis. In short, decision-makers are interested not simply in an isolated perspective nor in a smoothed-out mythical statistical average (as in the mythical family with 2.2 children) but in a wide and diverse range of experiences of a shared phenomenon (Pluye and Hong 2014). This phenomenon of interest could relate to perceptions and experiences of a particular health condition or, equally, attitudes towards a specific intervention. Patients' perspectives can be accessed via qualitative research. Context-sensitive primary qualitative research can be conducted to address a specific research question. However, the time and resources required to plan and conduct primary research can prove prohibitive. One alternative,

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used by many health technology agencies, is to harness the collective richness of multiple qualitative research studies within an evidence synthesis. Such an approach starts from an implicit assumption, contested by others, that qualitative research findings may be considered 'transferable' (Finfgeld-Connett 2010). In recent years qualitative evidence synthesis (QES) has therefore become a flexible vehicle for collecting and analysing the collective accounts of patients or health service users.

Why is 'qualitative evidence synthesis' the preferred term for what has been otherwise labelled as qualitative meta-syntheses or qualitative systematic reviews? In 2011 the co-conveners of the then Cochrane Qualitative Methods Group settled on this term to distance the emerging methodology from the dominant methods of systematic reviews of effects and to signal the potential of this group of methods for a wider range of types of evidence. So, future 'evidence' might use these same methods of synthesis to incorporate patients' perspectives from online bulletin boards or narrated patient real-life experiences collected by interview (Healthtalk 2016).

While the incorporation of more diverse types of evidence remains aspirational, the methodology of QES has enjoyed accelerated wide-scale development. In 1998 a landmark meta-synthesis sought to incorporate perspectives from 43 interpretive research reports of the lived experience of patients with diabetes (Paterson et al. 1998). The review team explicitly sought to extend 'the analysis of individual research studies beyond individual experience to incorporate dominant system beliefs and health system ideologies' (Paterson et al. 1998). This intent is shared by many current QES in seeking to produce a more nuanced understanding of how patients interact within the context of health services and the professionals and support staff who deliver those services.

15.2 Eliciting Patients' Perspectives in HTA

From their earliest years, QES in health care have offered a vehicle for otherwise disenfranchised patient groups (Warr 2004; Booth 2016). As Toye and colleagues observe: 'Affirming a person's experience and allowing an empathetic interpretation of their story is not an adjunct [i.e. optional extra], but integral to care' (Toye et al. 2013, p. e835). Recent examples of QES within an integrated HTA include those on male obesity (Robertson et al. 2014), teenage repeat pregnancy (Whitaker et al. 2016) and prevention of postnatal depression (Morrell et al. 2016). The last two of these HTA reports not only include qualitative synthesis components but extend to ask 'what works for whom under what circumstances' using a specific methodology, realist synthesis.

Health systems place increasing emphasis on the design and delivery of services that are 'patient focused' (Hansen et al. 2011). In response to this imperative, commissioners of HTA, and of health services research more generally, may commission a review team to undertake robust secondary research to understand the diverse experiences and perspectives of patients that can be assessed alongside quantitative evidence of clinical and cost-effectiveness to inform health policy and clinical

decision-making. In addition, QES addresses contemporary concerns about possible research waste.

Patient participation can also constitute a topic for QES in its own right. QES of patient participation has examined patients' feelings about ward nursing regimes (Alexander 2006), participation in nursing care on medical wards (Tobiano et al. 2015) and shared decision-making in palliative care (Bélanger et al. 2011).

Mixed methods approaches to synthesis remain in their infancy; mixed methods reviews may summarise quantitative (i.e. from a review of randomised controlled trials) and qualitative (i.e. within a QES) data separately and then seek to integrate the two types of evidence, or, alternatively, they may seek to review only mixed methods primary studies (Heyvaert et al. 2016). In an example of the former, Gagnon and colleagues have demonstrated that patient or public perspectives could add important dimensions to the evaluation of health technologies, while cautioning of a need for more systematic approaches to considering patient and public perspectives in HTA (Gagnon et al. 2009).

15.3 Choosing an ~Appropriate Method of QES

Seven factors are important when selecting an appropriate method of QES (Booth et al. 2016). These factors, identified from the literature, can be organised under the mnemonic RETREAT (**R**esearch Question, **E**pistemology, **T**ime, **R**esources, **E**xpertise, **A**udience and Purpose, **T**ypes of Data) (Box 15.1). These are considered in turn.

Box 15.1 Considerations When Selecting a Method of Qualitative Evidence Synthesis (RETREAT Mnemonic)

Review Question
Epistemology
Time
Resources
Expertise
Audience and Purpose
Type of Data

A key consideration when selecting a method of synthesis relates to the nature of the Research question [R]. Will the research question share the same scope as an associated effectiveness question, is it complementary or does it have a wider ambition? Observers comment on two particular characteristics of question formulation for qualitative reviews; first, the review question is more a 'compass' rather than the 'anchor' associated with effectiveness reviews (Dixon-Woods et al. 2006b). As the HTA review team follows up initial leads, they may unearth further lines of inquiry—in a similar

way to inquiry in primary qualitative research. Secondly, an HTA review team may be interested in qualitative data that extends beyond the experience of an intervention, particularly as a health technology may be novel and qualitative research scarce. The QES may have to examine patient experiences of a condition, both with and without any intervention, and may consequently be broader in scope than the effectiveness question (Lorenc et al. 2012). The case study on antimicrobial wound dressings in Chap. 27 is one such HTA example where the literature search had to be broadened beyond the original review question in recognition of a shortage of evidence. Where the QES shares broadly the same scope as an effectiveness question, the HTA review team can use an aggregative method of synthesis (e.g. meta-aggregation or thematic synthesis without theory generation). However, if an HTA review team seeks to explore qualitative aspects of an intervention at a more theoretical, conceptual level, then they may prefer the greater freedom offered by an interpretive method such as meta-ethnography.

Epistemological concerns [E] may hold comparatively lesser weight within a pragmatic health technology assessment than they do in a thesis or similar academic work. Is the type of knowledge being generated by the QES a generalisable theory, or is it to be confined to specific points for implementation? The HTA review team must stay sensitive to the epistemology that underpins each methodology when making a selection (Barnett-Page and Thomas 2009).

Of more immediate concern for a review team when selecting a method for performing a QES within the context of HTA is the triad of Time, Resources and Expertise [T,R,E]. HTAs are frequently conducted within severe time and resource constraints. HTA teams are commonly assembled from an existing pool of staff within an institution. Less ambitious, and more easily acquired, methods of synthesis, such as meta-aggregation, thematic synthesis and framework synthesis are more amenable to a rapid approach. Realist synthesis accommodates its own specific rapid variant—labelled rapid realist synthesis (Saul et al. 2013). As HTA agencies trade timeliness against rigour, a greater range of rapid QES variants is likely to be developed.

Key within an HTA context are considerations of Audience and purpose [A]. Decision-makers favour methods that yield a clear link between findings and subsequent recommendations. Barnett-Page and Thomas observe that ‘the output of some methods of synthesis (Thematic Synthesis, textual Narrative Synthesis, Framework Synthesis, and ecological triangulation) is more directly relevant to policymakers and designers of interventions than the outputs of methods with a more constructivist orientation…which are generally more complex and conceptual’ (Barnett-Page and Thomas 2009, p. 9).

A final logistical consideration relates to the Types of data [T] to be synthesised. Interpretive methods such as meta-ethnography require data that is conceptually rich and contextually thick. Where qualitative data sources offer minimal data, as in thin case study reports published in professional journals, the potential to undertake a more interpretive exploration is compromised. Thin data is unlikely to be able to sustain meta-ethnography. More superficial approaches, such as thematic synthesis, may be indicated as appropriate alternatives.

Given the potentially bewildering variety of choices, the most practical advice is for an HTA review team to settle for thematic synthesis where the topic is poorly theorised or where there is little consensus on prevailing theory. Thematic synthesis offers additional merit given that thematic synthesis is a precursor to meta-ethnography, and so this remains an open option should data prove rich and thick enough to sustain this more interpretive process. Alternatively, where a field is well theorised and one or more frameworks receive widespread recognition, this becomes an indication for choosing a framework synthesis (Dixon-Woods 2011). Further details on selection of an appropriate QES methodology within HTA are available from the free online INTEGRATE-HTA guidance on this topic (Booth et al. 2016).

15.4 Undertaking a Qualitative Evidence Synthesis

While great variety exists in the overall methods available for qualitative synthesis, Garside (2008) demonstrates that nine phases are common to most types of synthesis (Table 15.1). Individual methods vary in the precise sequencing of these phases and the degree of iteration required by each method.

The first phase of undertaking a QES parallels that for a quantitative systematic review in requiring (1) *development of a clearly formulated review question*. Whereas those conducting effectiveness reviews favour the PICO (Population, Intervention, Comparison, Outcomes) format, those conducting qualitative synthesis find it helpful to adopt a more relativist 'lens' (Stern et al. 2014). One question format that is gaining in popularity in QES is Setting, Perspective, phenomenon of Interest, Comparison, Evaluation (SPICE) (Riesenberg and Justice 2014).

For the above reasons, (2) *scoping* becomes a prerequisite second phase before undertaking the actual review itself. Such scoping may involve identification of

Table 15.1 Comparison of the phases of a qualitative evidence synthesis and a systematic review

	Qualitative evidence synthesis	Systematic review
1.	Development of clearly formulated review question	Formulate the problem
2.	Scoping the literature	
3.	Formal identification of the relevant literature	Literature search
4.	Initial assessment of study reports	
		Data extraction Critical appraisal of studies (quality assessment)
5.	Analysis and synthesis	
6.	Preliminary synthesis	
7.	Full synthesis	Data synthesis
8.	Dissemination	Presenting results (writing the report)
9.	Throughout the process	

'clusters' of related studies that can be forensically pursued in order to add thicker contextual detail and a richer conceptual understanding (Booth et al. 2013b). Data sources may include pilot studies, feasibility studies and process evaluations as well as 'sibling' qualitative studies that run alongside a higher-profile trial. Health Services Research PubMed Queries (https://www.nlm.nih.gov/nichsr/hedges/search.html) offers a rapid search facility for scoping qualitative research topics or related topics of appropriateness, process assessment or quality improvement, using a choice of either broad sensitive or narrow specific search filters.

Once the HTA review team has articulated the review question and set its conceptual, logistical and terminological limits, the team proceeds to (3) *formal identification of relevant literature* (Finfgeld-Connett and Johnson 2012). While determining the actual population of studies for inclusion is no less important than for an effectiveness systematic review, the underlying rationale may be markedly different. An effectiveness review seeks to minimise bias by assembling as comprehensive sample of the existing studies as resources allow. However, for a QES, an HTA review team wishes to gain a holistic understanding of the phenomenon of interest. The intent is configurative, rather than aggregative. To illustrate, an effectiveness review often seeks to demonstrate that an intervention is effective on average for a general population. For a qualitative synthesis, an HTA review team may be equally interested in those who find an intervention unacceptable or those who receive less than the expected benefit from the intervention. This interest in the 'disconfirming case' alongside other sources of variation opens up a full array of methods of sampling from qualitative research (Benoot et al. 2016; Suri 2011).

The unrivalled coverage of MEDLINE makes it a first port of call for most qualitative synthesis questions (Booth 2016). Admittedly retrieval of qualitative research often proves more challenging given such factors as limited indexing, non-indicative titles and abstracts (Dixon-Woods et al. 2006a) and the sheer predominance of quantitative studies. CINAHL, with its focus on literatures where qualitative research is more accepted together with its inclusion of theses and dissertations, is also considered a primary source (Subirana et al. 2005). EMBASE, PsycINFO, Sociological Abstracts and Social Sciences Citation Index (Web of Knowledge) also feature prominently in QES search methods. When searching the UK literature, these may be augmented by country-specific databases such as ASSIA and the British Nursing Index and the Index to Theses (Stansfield et al. 2012). This may be equally true for other geographic regions. Predesigned filters exist for retrieving qualitative research studies from the four main international databases: MEDLINE (Wong et al. 2004), EMBASE (Walters et al. 2006), CINAHL (Wilczynski et al. 2007) and PsycINFO (McKibbon et al. 2006). However, it may be equally useful to use hedges of key terms associated with a particular perspective or phenomenon such as patient involvement (Resource 2016b) or quality of life (Resource 2016a). In several cases, a short list of qualitative terms has been found to perform comparably to a more expansive list, possibly because multiple retrieval terms often occur in the same abstract (Flemming and Briggs 2007; Gorecki et al. 2010). However, this requires testing across a greater range of review topics and literatures.

Certainly, it is important not to rely too much on conventional subject searching of bibliographic databases but to use numerous supplementary techniques such as backward and forward citation searching, handsearching of relevant journals such as *The Patient*; *Health Expectations*; *Value in Health*; *Social Science and Medicine*; *Culture, Medicine, and Psychiatry*; *Research Involvement and Engagement*; *Anthropology and Medicine*; *and Sociology of Health and Illness* and contact with authors and experts (Papaioannou et al. 2010; Greenhalgh and Peacock 2005). Websites of national patient organisations may also yield useful information. The case study in Chap. 27 on antimicrobial wound dressing offers a good example where reliance on subject searches on bibliographic databases alone would have seriously degraded the HTA response.

The fourth phase involves (4) *an initial assessment of study reports*. After preliminary reading and re-reading, the QES team forms a picture of the literature and how it is structured. Theories, either explicitly stated or implicitly referenced, start to become apparent (Booth and Carroll 2015b). Such conceptual frameworks may become a useful vehicle for data extraction through framework synthesis (Booth and Carroll 2015a).

Next, (5) *analysis and synthesis* takes place. Constant comparison is used to identify patterns and similarities across reports. Refutational findings must be reconciled (Booth et al. 2013a). At this point quality assessment may be undertaken, either using a single generic assessment tool or checklist or a battery of checklists designed for individual types of study (Carroll and Booth 2015). The review team considers the extent to which the synthesis and its findings are based on robust qualitative studies (Carroll et al. 2012).

Preliminary synthesis (6) involves organisational procedures such as categorising, tabulation and the creation of mind maps. The review team explores relationships both within and between studies. *Full synthesis* (7) may, in its simplest form, be achieved through a process of thematic synthesis or, with greater interpretive complexity, through translation of concepts and metaphors as undertaken for meta-ethnography. Meta-ethnography seeks to interpret studies rather than simply aggregating them, with the intent being to generate a new theory or 'line of argument' to explain all the studies (France et al. 2014).

Considerations of the intended audience subsequently inform the methods chosen for (8) *dissemination*. Exploratory methods of presentation include idea maps and concept maps (Popay et al. 2006). The review team assesses the strengths and limitations of the review itself and of the body of included studies. Optimally, all stakeholders are consulted so that emerging findings become an organic product of knowledge co-creation. However, it is not unexpected to find that stakeholders are not able to recognise the synthetic findings from the interpretive process in their entirety as they often possess only a fragmented, yet valid, perspective. Essentially, therefore, a review team is substituting the authenticity of a single participant's view of the phenomenon with a more overarching interpretive account that attempts to identify and reconcile multiple perspectives.

Throughout the process (9), the multidisciplinary team brings together their different perspectives not for consensus, as is the case for multiple reviewers in an effectiveness

review, but more for divergence and interpretive richness (Booth et al. 2013a). Reflexivity, the facility of qualitative researchers to consider the impact of their own role as researchers on the synthetic process and resultant product, is surfaced and discussed (Newton et al. 2012). Notwithstanding the iterative and recursive nature of the qualitative synthesis, it shares the requirement of systematic reviews more generally to document methods and decisions to increase confidence in the findings (Benoot et al. 2016).

Recent years have seen attention focused on an additional stage in the QES process, to make such reviews even more comparable to effectiveness reviews, namely, the production of assessments of qualitative findings. The GRADE-CERQual subgroup has developed a four-component approach that assesses individual review findings for adequacy, coherence, methodological limitations and relevance (Lewin et al. 2015). Assessments of the findings from a QES are designed to parallel the strength of findings tables produced for GRADE assessments, whereby quantitative findings have previously been assessed against four corresponding components. Limited examples exist of the use of this CERQual approach within current HTA processes (Morrell et al. 2016; Whitaker et al. 2016), but proof of concept has been demonstrated for Cochrane and WHO systematic reviews.

15.5 Integration of Quantitative and Qualitative Data

Finally, integration of quantitative and qualitative evidence allows a team to produce evidence products to inform complex HTA problems. Approaches to integrating patients' perspectives with effectiveness data can utilise one or more of seven potential mechanisms:

1. *Use a review methodology designed to handle both quantitative and qualitative data* (i.e. integration at a methods level). Realist synthesis seeks to identify and then explore configurations of context, mechanism and outcomes for those circumstances under which an intervention or programme is likely to work well and those under which it may perform suboptimally (Rycroft-Malone et al. 2012). The HTA review team may also extract such configurations from the introductory or discussion sections of randomised controlled trials or from qualitative or process evaluation data. Critical interpretive synthesis reviews a purposively sampled selection of literature to examine how the literature has problematised a particular phenomenon. More broadly meta-narrative review examines how a particular concept has been characterised within different paradigms and disciplines. Essentially all three methodologies seek to reconcile the quantitative and qualitative literatures within an overarching narrative.
2. *Use an external conceptual framework*, typically identified from a parallel search process specifically for theory, as a structure by which to bring together qualitative and quantitative data. This framework may be specific to the topic of the review, may be a 'best-fit' framework that matches against several critical characteristics of the topic or may be a meta-framework that fuses together multiple models or frameworks (Booth and Carroll 2015a).

3. *Use an internally generated framework* derived from consultation with stakeholders (Oliver et al. 2008) or a simple matrix that places themes from the qualitative literature alongside outcome domains from the quantitative studies (Candy et al. 2011; Millar et al. 2012).
4. *Use a programme theory*, also an essential feature of the realist synthesis methodology in (1) above, against which the review team maps various features of the quantitative and qualitative literature to 'chains' of causation.
5. Related to (4) above, *construct a logic model* as a framework against which data is mapped and then analysed. This is an atheoretical variant of the framework method in (2) above (Baxter et al. 2014).
6. *Perform subgroup analyses* to bring quantitative and qualitative data together for particular subgroups.
7. *Use quantitative and qualitative techniques sequentially*, rather than in parallel. For example, Bayesian synthesis uses qualitative evidence to identify important factors associated with an intervention and then the quantitative evidence to explore their relative effects (Roberts et al. 2002). Alternatively, qualitative comparative analysis involves using truth tables to explore the internal logic by which factors identified qualitatively may exert an influence, as presented in the quantitative data (Thomas et al. 2014; Brunton et al. 2014).

Many methods for integrating quantitative and qualitative data remain tentative with few worked examples, and a considerable agenda persists for empirical testing. Currently, when QES has been undertaken in an HTA, the HTA report generally includes the QES as a separate stand-alone chapter thus sidestepping methodological difficulties. Nevertheless, it is clear that considerable potential for enhanced integration of quantitative and qualitative data exists, and this remains a major methodological challenge over the next few years.

15.6 Discussion

As is apparent from the above consideration, qualitative evidence synthesis is one of the fastest growing areas of research synthesis methodology. Particular drivers for this growth include increased recognition of the complexity of decision problems and increasing acknowledgement of the complexity of many human-mediated technologies. Both of these drivers are particularly relevant in the context of patient involvement in HTA. Valuing the patient experience requires incorporation of patient values and perspectives in the decision-making process. Recognising that the effectiveness of many health technologies is mediated by multiple factors related to the patient-clinician interaction makes it critical to explore such relationships more thoroughly.

Not to be overlooked is a vital role that patient and public involvement can play in improving the design and analysis of qualitative evidence syntheses, as for systematic reviews more generally (Harris et al. 2015; Boote et al. 2012; Oliver et al. 2015). Many considerations regarding the timing, extent and nature of patient involvement in HTA are shared by quantitative and qualitative systematic reviews alike.

As with other methods of synthesis, QES is limited by the quality of reporting of primary studies. Further limitations relate to whether the primary research questions of the included studies map exactly to the review question or whether the primary studies only yield incidental insights. For many commentators, particularly those who are more familiar with the quantitative paradigm, the degree of interpretation is a source of discomfort – the HTA review team is in effect offering interpretations (by the team) of interpretations (by the primary authors) of the experiences and perspectives of research participants.

Innovations in grading of recommendations using the GRADE-CERQual system for qualitative evidence syntheses (Lewin et al. 2015), envisaged as comparable to, and potentially integrated with, the GRADE system for effectiveness studies, offer further opportunities for incorporation of synthesised patients' perspectives within HTAs, health-care policy and decision-making.

References

Alexander J. Patients' feelings about ward nursing regimes and involvement in rule construction. J Psychiatr Ment Health Nurs. 2006;13:543–53.

Barnett-Page E, Thomas J. Methods for the synthesis of qualitative research: a critical review. BMC Med Res Methodol. 2009;9:59.

Baxter SK, Blank L, Woods HB, Payne N, Rimmer M, Goyder E. Using logic model methods in systematic review synthesis: describing complex pathways in referral management interventions. BMC Med Res Methodol. 2014;14:62.

Bélanger E, Rodríguez C, Groleau D. Shared decision-making in palliative care: a systematic mixed studies review using narrative synthesis. Palliat Med. 2011;25:242–61.

Benoot C, Hannes K, Bilsen J. The use of purposeful sampling in a qualitative evidence synthesis: a worked example on sexual adjustment to a cancer trajectory. BMC Med Res Methodol. 2016;16:21.

Boote J, Baird W, Sutton A. Involving the public in systematic reviews: a narrative review of organizational approaches and eight case examples. J Compar Eff Res. 2012;1:409–20.

Booth A. Searching for qualitative research for inclusion in systematic reviews: a structured methodological review. Syst Rev. 2016;5:74.

Booth A, Carroll C. How to build up the actionable knowledge base: the role of 'best fit' framework synthesis for studies of improvement in healthcare. BMJ Qual Saf. 2015a;24:700–8.

Booth A, Carroll C. Systematic searching for theory to inform systematic reviews: is it feasible? Is it desirable? Health Info Libr J. 2015b;32:220–35.

Booth A, Carroll C, Ilott I, Low LL, Cooper K. Desperately seeking dissonance: identifying the disconfirming case in qualitative evidence synthesis. Qual Health Res. 2013a;23:126–41.

Booth A, Harris J, Croot E, Springett J, Campbell F, Wilkins E. Towards a methodology for cluster searching to provide conceptual and contextual "richness" for systematic reviews of complex interventions: case study (CLUSTER). BMC Med Res Methodol. 2013b;13:118.

Booth A, Noyes J, Flemming K, Gerhardus A, Wahlster P, Van Der Wilt GJ, et al. Guidance on choosing qualitative evidence synthesis methods for use in health technology assessments of complex interventions. INTEGRATE-HTA. 2016. Available: http://www.integrate-hta.eu/wp-content/uploads/2016/02/Guidance-on-choosing-qualitative-evidence-synthesis-methods-for-use-in-HTA-of-complex-interventions.pdf. Accessed 8 Apr. 2016.

Brunton G, O'Mara-Eves A, Thomas J. The 'active ingredients' for successful community engagement with disadvantaged expectant and new mothers: a qualitative comparative analysis. J Adv Nurs. 2014;70:2847–60.

Candy B, King M, Jones L, Oliver S. Using qualitative synthesis to explore heterogeneity of complex interventions. BMC Med Res Methodol. 2011;11:124.
Carroll C, Booth A. Quality assessment of qualitative evidence for systematic review and synthesis: is it meaningful, and if so, how should it be performed? Res Synth Methods. 2015;6:149–54.
Carroll C, Booth A, Lloyd-Jones M. Should we exclude inadequately reported studies from qualitative systematic reviews? An evaluation of sensitivity analyses in two case study reviews. Qual Health Res. 2012;22:1425–34.
Dixon-Woods M. Using framework-based synthesis for conducting reviews of qualitative studies. BMC Med. 2011;9.
Dixon-Woods M, Bonas S, Booth A, Jones D, Miller T, Sutton A, et al. How can systematic reviews incorporate qualitative research? A critical perspective. Qual Res. 2006a;6:27–44.
Dixon-Woods M, Cavers D, Agarwal S, Annandale E, Arthur A, Harvey J, et al. Conducting a critical interpretive synthesis of the literature on access to healthcare by vulnerable groups. BMC Med Res Methodol. 2006b;6:35.
Finfgeld-Connett D. Generalizability and transferability of meta-synthesis research findings. J Adv Nurs. 2010;66:246–54.
Finfgeld-Connett D, Johnson ED. Literature search strategies for conducting knowledge-building and theory-generating qualitative systematic reviews. J Adv Nurs. 2012;69:194–204.
Flemming K, Briggs M. Electronic searching to locate qualitative research: evaluation of three strategies. J Adv Nurs. 2007;57:95–100.
France EF, Ring N, Thomas R, Noyes J, Maxwell M, Jepson R. A methodological systematic review of what's wrong with meta-ethnography reporting. BMC Med Res Methodol. 2014;14:119.
Gagnon M-P, Lepage-Savary D, Gagnon J, St-Pierre M, Simard C, Rhainds M, et al. Introducing patient perspective in health technology assessment at the local level. BMC Health Serv Res. 2009;9:54.
Garside R. A comparison of methods for the systematic review of qualitative research : two examples using meta-ethnography and meta-study. Dissertation, Universities of Exeter and Plymouth. 2008.
Gorecki CA, Brown JM, Briggs M, Nixon J. Evaluation of five search strategies in retrieving qualitative patient-reported electronic data on the impact of pressure ulcers on quality of life. J Adv Nurs. 2010;66:645–52.
Greenhalgh T, Peacock R. Effectiveness and efficiency of search methods in systematic reviews of complex evidence: audit of primary sources. BMJ. 2005;331:1064–5.
Hansen HP, Draborg E, Kristensen FB. Exploring qualitative research synthesis: the role of patients' perspectives in health policy design and decision making. Patient. 2011;4:143–52.
Harris J, Croot L, Thompson J, Springett J. How stakeholder participation can contribute to systematic reviews of complex interventions. J Epidemiol Community Health. 2015;70:207–14.
Healthtalk. http://www.healthtalk.org/ (2016). Accessed 23 Nov 2016.
Heyvaert M, Hannes K, Onghena P. Using mixed methods research synthesis for literature reviews. Mixed methods research series, vol. 4. London: Sage; 2016.
InterTASC Information Specialists' Sub-Group (ISSG) Filters to Identify Studies of Patient Views and Patient Experiences [online]. [Online]. Sites.google.com: InterTASC Information Specialists' Sub-Group (ISSG) Information Specialists' Sub-Group (ISSG). 2016b. Available: https://sites.google.com/a/york.ac.uk/issg-search-filters-resource/filters-to-identify-studies-of--public-views-and-patient-issues. Accessed 8 Apr 2016.
InterTASC Information Specialists' Sub-Group (ISSG). Filters to Identify Quality of Life Studies [online]. [Online]. Sites.google.com: InterTASC Information Specialists' Sub-Group (ISSG) Information Specialists' Sub-Group (ISSG). 2016a. Available: https://sites.google.com/a/york.ac.uk/issg-search-filters-resource/filters-to-identify-quality-of-life-studies [Accessed 8 Apr. 2016 2016].
Lewin S, Glenton C, Munthe-Kaas H, Carlsen B, Colvin CJ, Gülmezoglu M, et al. Using qualitative evidence in decision making for health and social interventions: an approach to assess confidence in findings from qualitative evidence syntheses (GRADE-CERQual). PLoS Med. 2015;12:e1001895.

Lorenc T, Pearson M, Jamal F, Cooper C, Garside R. The role of systematic reviews of qualitative evidence in evaluating interventions: a case study. Res Synth Methods. 2012;3:1–10.
McKibbon KA, Wilczynski NL, Haynes RB. Developing optimal search strategies for retrieving qualitative studies in PsycINFO. Eval Health Prof. 2006;29:440–54.
Millar R, Powell M, Dixon A. What was the programme theory of New Labour's Health System Reforms? J Health Serv Res Policy. 2012;17:Suppl 1:7–15.
Morrell CJ, Sutcliffe P, Booth A, Stevens J, Scope A, Stevenson M, et al. A systematic review, evidence synthesis and meta-analysis of quantitative and qualitative studies evaluating the clinical effectiveness, the cost-effectiveness, safety and acceptability of interventions to prevent postnatal depression. Health Technol Assess. 2016;20(37):1–414.
Newton B, Rothlingova Z, Gutteridge R, Lemarchand K, Raphael J. No room for reflexivity? Critical reflections following a systematic review of qualitative research. J Health Psychol. 2012;17:866–85.
Oliver K, Rees R, Brady LM, Kavanagh J, Oliver S, Thomas J. Broadening public participation in systematic reviews: a case example involving young people in two configurative reviews. Res Synth Methods, 2015;6:206–17.
Oliver SR, Rees RW, Clarke-Jones L, Milne R, Oakley AR, Gabbay J, et al. A multidimensional conceptual framework for analysing public involvement in health services research. Health Expect. 2008;11:72–84.
Papaioannou D, Sutton A, Carroll C, Booth A, Wong R. Literature searching for social science systematic reviews. Health Inf Libr J. 2010;27:114–22.
Paterson BL, Thorne S, Dewis M. Adapting to and managing diabetes. Image J Nurs Sch. 1998;30:57–62.
Pluye P, Hong QN. Combining the power of stories and the power of numbers: mixed methods research and mixed studies reviews. Annu Rev Public Health. 2014;35:29–45.
Popay J, Roberts H, Sowden A, Petticrew M, Arai L. Guidance on the conduct of narrative synthesis in systematic reviews. A product from the ESRC methods programme. Lancaster: Institute for Health Research; 2006.
Riesenberg LA, Justice EM. Conducting a successful systematic review of the literature, part 1. Nursing. 2014;44:13–7.
Ring N, Jepson R, Ritchie K. Methods of synthesizing qualitative research studies for health technology assessment. Int J Technol Assess Health Care. 2011a;27:384–90.
Ring N, Ritchie K, Mandava L, Jepson R. A guide to synthesising qualitative research for researchers undertaking health technology assessments and systematic reviews. NHS Quality Improvement Scotland; 2011b. Available: http://www.nhshealthquality.org/nhsqis/8837.html. Accessed 8 Apr 2016.
Roberts KA, Dixon-Woods M, Fitzpatrick R, Abrams KR, Jones DR. Factors affecting uptake of childhood immunisation: a Bayesian synthesis of qualitative and quantitative evidence. Lancet. 2002;360:1596–9.
Robertson C, Archibald D, Avenell A, Douglas F, Hoddinott P, Van Teijlingen E, et al. Systematic reviews of, and integrated report, on the quantitative, qualitative and economic evidence base for the management of obesity in men. Health Technol Assess. 2014;18(35):1–424.
Rycroft-Malone J, McCormack B, Hutchinson AM, Decorby K, Bucknall TK, Kent B, et al. Realist synthesis: illustrating the method for implementation research. Implement Sci. 2012;7:33.
Saul JE, Willis CD, Bitz J, Best A. A time-responsive tool for informing policy making: rapid realist review. Implement Sci. 2013;8:103.
Stansfield C, Kavanagh J, Rees R, Gomersall A, Thomas J. The selection of search sources influences the findings of a systematic review of people's views: a case study in public health. BMC Med Res Methodol. 2012;12.
Stern C, Jordan Z, McArthur A. Developing the review question and inclusion criteria. Am J Nurs. 2014;114:53–6.
Subirana M, Sola I, Garcia JM, Gich I, Urrutia G. A nursing qualitative systematic review required MEDLINE and CINAHL for study identification. J Clin Epidemiol. 2005;58:20–5.

Suri H. Purposeful sampling in qualitative research synthesis. Qual Res J. 2011;11:63–75.
Thomas J, O'Mara-Eves A, Brunton G. Using qualitative comparative analysis (QCA) in systematic reviews of complex interventions: a worked example. Syst Rev. 2014;3:67.
Tobiano G, Marshall A, Bucknall T, Chaboyer W. Patient participation in nursing care on medical wards: an integrative review. Int J Nurs Stud. 2015;52:1107–20.
Toye F, Seers K, Allcock N, Briggs M, Carr E, Andrews J, et al. Patients' experiences of chronic non-malignant musculoskeletal pain: a qualitative systematic review. Br J Gen Pract. 2013;63:e829–41.
Walters LA, Wilczynski NL, Haynes RB. Developing optimal search strategies for retrieving clinically relevant qualitative studies in EMBASE. Qual Health Res. 2006;16:162–8.
Warr DJ. Stories in the flesh and voices in the head: reflections on the context and impact of research with disadvantaged populations. Qual Health Res. 2004;14:578–87.
Whitaker R, Hendry M, Aslam R, Booth A, Carter B, Charles JM, et al. Intervention now to eliminate repeat unintended pregnancy in teenagers (INTERUPT): a systematic review of intervention effectiveness and cost-effectiveness, and qualitative and realist synthesis of implementation factors and user engagement. Health Technol Assess. 2016;20:1–214.
Wilczynski NL, Marks S, Haynes RB. Search strategies for identifying qualitative studies in CINAHL. Qual Health Res. 2007;17:705–10.
Wong SSL, Wilczynski NL, Haynes RB. Developing optimal search strategies for detecting clinically relevant qualitative studies in MEDLINE. MedInfo 2004: Proceedings of the 11th World Congress on Medical Informatics, Pt 1 and 2. 2004;107: 311–14.

Chapter 16
Evaluation of Patient Involvement in HTA

Marie-Pierre Gagnon, Mylène Tantchou Dipankui, and Deirdre DeJean

16.1 Introduction

This book outlines a range of goals of patient involvement in HTA and expected benefits. However, the benefits remain hypothetical in the absence of formal evaluation of patient involvement in HTA. This chapter aims to provide an overview of current practices regarding the evaluation of patient involvement in HTA. The first part posits the need for evaluating patient involvement in HTA and presents some examples of how it could impact HTA. Then, the second part presents current evidence on the impact of patient involvement in fields related to HTA, such as clinical research and clinical guideline development. The third part focuses on some of the main gaps identified in the literature, and the fourth part highlights challenges related to evaluating patient involvement in HTA, including conceptual, methodological and practical aspects. The chapter concludes by proposing directions for supporting patient involvement practices in HTA and providing guidance to ensure rigorous evaluations of the impact of patient involvement in HTA that are adapted to each context.

Currently, there is a scarcity of rigorous evaluations of patient involvement initiatives in HTA. In a survey of 33 HTA organisations, 22 (67%) indicated that patients were involved in their activities, but among these, only 4 (19%) had evalu-

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ated patient involvement (Hailey et al. 2013). These results are confirmed by other surveys conducted among decision-makers and patient organisations in Europe (European Patients' Forum 2013) showing that few HTA bodies had a structured approach to involving patients in their activities and limited formal evaluation of the impact of this involvement. Evaluating patient involvement initiatives is thus crucial to strengthening the evidence base supporting the value that patient involvement brings to the HTA process and products, as well as its impact on decisions regarding health technologies and services, and ultimately on health outcomes. Evaluations of patient involvement in HTA are also needed to improve the way that patient involvement is done and ensure it adheres to best practices.

16.2 Need for Evaluating Patient Involvement in HTA

In recent years, public and patient involvement has emerged as an imperative for more informed, transparent, accountable and legitimate decisions about health technologies (Abelson et al. 2007; Facey et al. 2010; Boivin et al. 2014; Gagnon et al. 2011). However, evidence from systematic reviews of patient involvement in HTA is scarce. A recent literature review of 18 studies shows major variation between HTA organisations with respect to patient involvement, with most of them seeking limited patient input through consultation (Hicks et al. 2014).

A previous systematic review of 24 studies (Gagnon et al. 2011) identified two main ways to involve patients or public in HTA. First, patients or their representatives are studied in research in order to generate evidence about their perspectives, experiences or preferences about a health technology or a clinical intervention. The second way is direct participation of patients in one or several steps of the HTA process: topic identification and selection, prioritisation, formulation and scoping of the evaluation question, evidence assessment and dissemination and implementation of HTA recommendations.

Evaluation is required to examine the process and impact of patient involvement in HTA from different stakeholders' perspectives (patients, healthcare providers, managers, policy-makers), but few studies have examined this to date (Gagnon et al. 2011; Hansen et al. 2011; Gauvin et al. 2014). Furthermore, the lack of common frameworks and methodologies for patient involvement in HTA impedes comparison of patient involvement initiatives (Hicks et al. 2014). Although several models have been proposed to describe the types and levels of patient involvement in HTA or in other related fields (Gauvin et al. 2010; Esmail et al. 2015; Gagnon et al. 2015), it is difficult to identify which strategies are the most suitable for a specific HTA topic or in a particular context. In addition, the rationale for evaluating patient involvement in HTA but not that of other stakeholder groups, such as managers, healthcare providers or health technology developers, is unclear. A key argument against evaluating patient involvement is that as payers and users of healthcare, patients have the right to participate in decisions related to services provided and

therefore should be involved regardless of the impact (Staley 2015). Thus, there is a moral obligation to involve patients as the ultimate end users of HTA (Esmail et al. 2015). Nevertheless, Staley (2015) suggests that evaluating patient involvement remains essential in order to improve how it is done and to ensure it has the desired impact, which could vary depending on the specific context and purpose of patient involvement.

To date, few patient involvement activities in HTA have been formally evaluated. A systematic review on patient and public involvement in HTA (Gagnon et al. 2011) found only nine studies that addressed the influence of patient consultation on the definition of aspects that should be considered for assessing the value of a given technology, treatment or intervention. Most of these studies indicated that consulting patients brings important dimensions to the evaluation of technologies and clinical interventions that could differ from those considered by clinicians. For instance, Kinter et al. (2009) found that incorporating patient-relevant endpoints into the evaluation of a treatment for schizophrenia brought crucial dimensions not covered by traditional clinical measures. Another example of how patient involvement has influenced HTA is found in the study by van Kammen et al. (2006) that assessed the influence of consulting patient groups on recommendations about subfertility care. The study showed that patient organisations found the new scientific evidence gained from this HTA very useful. However, it remains difficult to estimate the effect of patient involvement in HTA from the studies included in this review because of their heterogeneity.

16.3 Value of Patient Involvement: Evidence from Other Fields

There is growing evidence of the value of patient involvement in other fields related to healthcare decision-making, such as clinical guideline development (Ham et al. 2015), healthcare priority setting (Boivin et al. 2014) and health research (Brett et al. 2014; Wilson et al. 2015). In the field of health communication, a Cochrane systematic review (Nilsen et al. 2006) found some evidence to support the effectiveness of involving users in the development of patient information material. The results indicate that material produced with input from patient representatives is perceived as more relevant, readable and understandable to patients.

Ham et al. (2015) evaluated patient involvement in the development of clinical practice guidelines on employment and severe mental illness using a monitoring and evaluation framework. Their framework comprised two main categories: the process of patient involvement in guideline development and the outcomes of their involvement. The findings indicate that the patient involvement process was supported through the use of different methods for getting patient input, the reflection of patient input in the guideline, a supportive attitude among professionals and

attention to patient involvement throughout the process. However, some limitations were highlighted such as the representativeness of patients involved, the articulation of the patient perspective in the guideline and the transparency regarding the methods of involvement (*ibid*.).

Based on a review of current or recent research projects related to six healthcare areas that were undertaken in England, Wilson et al. (2015) conducted a realist evaluation of patient and public involvement in research. They highlighted six elements characterising effective initiatives that could apply to patient involvement in HTA, as presented in Box 16.1. These elements also echo the enablers for patient participation in HTA presented in Chap. 5.

Box 16.1 Key elements for Effective Patient Involvement in HTA Based on Wilson et al. (2015)

1. Shared understanding of the purposes of involvement
2. Key individual coordinating the involvement process
3. Diversity of people represented
4. HTA team that is supportive of patient and public involvement
5. Relationships that were established and maintained over time
6. Systematic and proactive evaluation of involvement

16.4 Main Gaps in the Evaluation of Patient Involvement in HTA

This section identifies the main gaps that authors have reported in the literature on patient involvement in HTA.

16.4.1 Lack of Validated Frameworks of the Process and Results of Patient Involvement

The vast majority of studies evaluating patient involvement in HTA are carried out in the absence of an evaluation framework that would inform the choice of evaluation criteria. In that sense, a logic model could be useful, as it provides an organised representation of the different components of the intervention and the relationships between them. This also relates to the lack of frameworks specific to patient involvement in HTA that could support the articulation of its goals. For instance, the mosaic of patient participation in HTA presented in Chap. 5 provides a comprehensive framework to guide patient involvement practices and could also inform the selection of criteria and indicators for evaluating these practices. Thus, for a meaningful evaluation of patient involvement in HTA, it has to be clear what the goals are and from whose perspective (Chap. 3).

In addition, there is a lack of clearly identified relationships among the process and outcome variables of interest. These gaps are not specific to HTA but are common in the literature on patient and public involvement in the health field in general. For instance, Abelson et al. (2010) found that much of the empirical public engagement evaluation work is done without any framework that defines the theoretical basis for the engagement process or the relationships among involvement mechanism and process or outcome variables of interest.

The framework proposed by Oliver et al. (2008) highlights key features that could influence the process and outcome of patient involvement in HTA. It considers the type of patients involved (individuals or members of consumer groups), the initiator of the involvement process, the degree of involvement (patients are consulted, collaborate or control the process), the choice of deliberation methods (qualitative or quantitative methods) and the methods for eliciting and aggregating values (such as voting, scoring or ranking). However, this framework does not include indicators of the outcomes and impact of patient involvement strategies.

Dipankui et al. (2015) developed a framework to assess patient involvement in HTA inspired by logic models used in programme evaluation to illustrate the relationships between the resources, activities, outputs and outcomes of a programme (Fig. 16.1). This framework incorporates three main components: (1) key elements of a logic model (Kellogg Foundation 2004), (2) evaluation criteria of patient involvement in HTA based on a general framework for evaluating public involvement (Rowe and Frewer 2005) and (3) contextual factors highlighted by Abelson et al. (2010). This framework was applied to the evaluation of patient involvement in the assessment of alternative measures to restraint and seclusion in psychiatry and in long-term care facilities for the elderly (Dipankui et al. 2015). As a generic framework, it considers most of the dimensions proposed in other frameworks for evaluating patient and public involvement in health decisions and could be used by HTA organisations to evaluate patient involvement in their activities. However, more studies applying this framework or others are needed in HTA in order to build a stronger evidence base on the process and outcomes of patient involvement and to identify the strengths and weaknesses of evaluation frameworks.

16.4.2 Lack of Clear Definition of Concepts

The literature on patient and public involvement in general provides some criteria against which involvement mechanisms are evaluated, and there have been some attempts to use a definition of ‘effectiveness’ as a starting point (Abelson et al. 2010). This echoes the situation in the field of HTA where some evaluation criteria are specified but often without distinguishing those related to the results and those related to the process. Therefore, it is difficult to know if the evaluation exercise conducted was an evaluation of the process or an evaluation of the outcomes. It is thus essential to underscore the role that evaluation plays in the current efforts to increase conceptual clarification in the field of patient involvement in HTA.

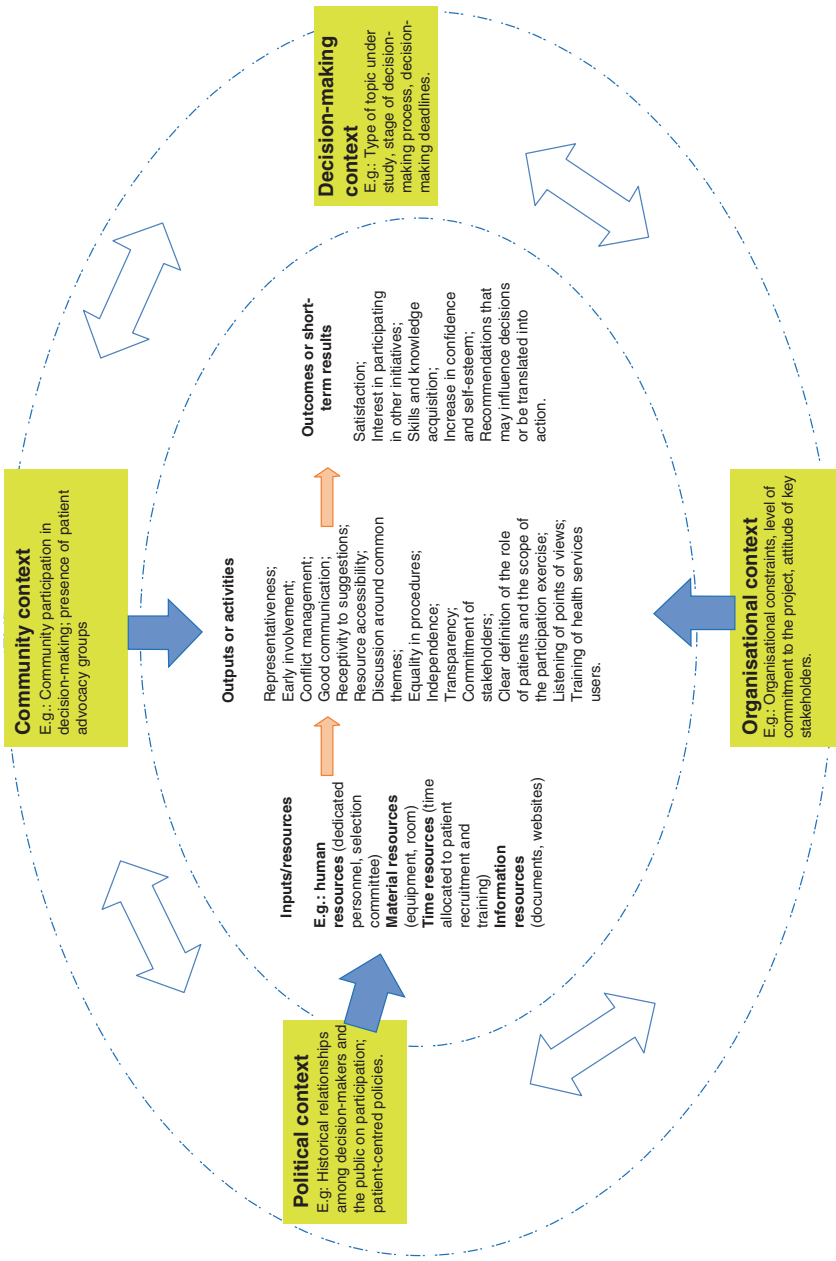

Fig. 16.1 Conceptual framework to evaluate patient involvement in health technology assessment

Clearly defining each of the concepts used also allows for more rigorous evaluation of patient involvement in the field of HTA. For instance, most authors do not specify what they mean by impact, and in general, when they talk about impacts, they describe short-term outcomes. Short-term outcomes should be attainable within 1–3 years, while longer-term outcomes should be achievable within a 4- to 6-year timeframe and should be reflected in impact occurring within about 7–10 years (Kellogg Foundation 2004). Difficulty arises in defining what is meant by impact in HTA (Wortley et al. 2015), and although most of the studies use the term 'impact', it would be more appropriate to talk about short- or medium-term outcomes. In this sense, measuring 'impact' seems unsuitable, and it is likely not adapted to the specific goals of involving patients in HTA. Thus, talking about value of patient involvement in HTA could be more appropriate.

16.4.3 Lack of Consideration of the Context

Another difficulty arises in the consideration of the context of studies of patient involvement in HTA. In fact, a growing number of researchers working in the field of patient and public involvement are recognising the significant role played by contextual variables that lie outside of the involvement process. According to Brett et al. (2014), the context refers to the environment in which involvement is undertaken and considers whether the right conditions are in place for patient involvement to work. Contextual variables can include funding, policy, physical environment or the attitude of those involved. With respect to HTA, Saarni et al. (2011) note that technologies and HTA are not executed in a vacuum, but always in a social and value context. These factors can have a significant shaping effect on how participants and policy-makers view the involvement initiative, and how it is implemented.

16.4.4 Different Methods and Techniques Used to Solicit Patient Evidence

The research methodologies outlined in Part II indicate how patients' perspectives and experiences can be studied in a robust scientific manner. Systematic approaches are now needed to agree how this patient-based evidence and patient input that is submitted directly by patients should be used in HTA.

The variety of approaches that are employed to obtain patient-based evidence and patient input in HTA challenges our ability to compare different patient involvement strategies. At the moment, there is very limited research comparing the effectiveness of different approaches for evaluating patient involvement in HTA (Gagnon et al. 2015). However, a 'one size fits all' approach is probably not the best option, and certain evaluation designs could be more appropriate, depending on the particular purpose of patient involvement in HTA.

In conclusion, four main gaps related to the evaluation of patient involvement in HTA could be identified from the literature:

1. Lack of validated framework to evaluate patient involvement
2. Lack of clear definition of concepts
3. Lack of consideration of the context
4. Differences in methods and techniques used to undertake patient involvement in HTA

These gaps lead us to identify the most common challenges to evaluating patient involvement in HTA and to propose strategies that could be used in order to answer these challenges. These challenges are listed in the following section.

16.5 Challenges Related to Evaluating Patient Involvement in HTA

The main challenges identified regarding the evaluation of patient involvement in HTA are grouped in conceptual, methodological and practical challenges. These challenges also offer avenues to consider for developing the field of patient involvement in HTA and thus strengthen its value.

16.5.1 Conceptual Challenges

Brett et al. (2014) report that patient involvement constitutes a complex intervention that requires appropriate evaluation. However, some preliminary steps are necessary before conducting the evaluation of patient involvement in a HTA. First, we need to use a common terminology when referring to key evaluation concepts such as process, outcomes, impact or effectiveness. The type of evaluation conducted and its purpose should also be clarified. Defining the goals of the evaluation is also an important prerequisite. Researchers wishing to design evaluation studies must first address questions such as: *Why is an evaluation being done? What will be done with the results? Who wants these results and how will they be used?*

The second conceptual challenge relates to the terms used when referring to patient involvement in HTA. For instance, the terms ‘patient’, ‘consumer’ or ‘service user’ are often used interchangeably in the literature. Chapter 3 reflects on these terms and considers the different roles of these individuals.

A third conceptual challenge is the development and/or adaptation of theoretical and conceptual frameworks for guiding the evaluation of patient involvement in HTA, and their validation in different contexts. Some existing frameworks can offer a comprehensive and integrated approach, although they are not developed specifically for this field.

For instance, the framework proposed by Esmail et al. (2015) for evaluating patient involvement in research can be used as a starting point to identify relevant dimensions related to the process, context and impact of patient involvement in HTA.

Table 16.1 Dimensions to consider in the evaluation of patient involvement in HTA

Dimensions related to the context	Dimensions related to the process	Dimensions related to the impact
Adequate resources and funding	Representativeness and diversity of patients involved	Patient empowerment
Attributes of the sponsoring organisation	Access to sufficient and adequate resources to facilitate patient engagement	Dissemination, translation and uptake of the results
Characteristics of the patients involved	Patient satisfaction with their engagement	Democracy and accountability
Availability of training for patients and researchers	Timing and frequency of engagement	Moral and ethical considerations
Nature of the topic being deliberated	Fairness and transparency of the process	
Time allocation	Definition of the patients' role and the purpose of involvement Adequate strategy in relation to the desired level of involvement	

Adapted from Esmail et al. (2015)

Table 16.1 shows some of the dimensions of this framework in relation to the context, process and impact of patient involvement that could apply to the field of HTA.

The framework presented in Fig. 16.1, developed by Dipankui et al. (2015), was specifically adapted to assess patient involvement in HTA but has only been applied in one specific context. It also considers dimensions related to the process of patient involvement in HTA and the context in which this involvement takes place. This framework uses a logic model to map the different types of outcomes related to patient involvement and distinguishes short- and medium-term results from long-term impact, acknowledging the importance of the evaluation timeframe.

16.5.2 Methodological Challenges

The diversity of study designs used to implement patient involvement in HTA makes it difficult to compare any involvement mechanism to another. Thus, several methodological challenges have to be addressed, from the selection of the most appropriate design for evaluating a specific patient involvement initiative to the consideration of results generalisability and theoretical/conceptual contribution. From the actual literature on patient involvement in HTA, there are no recommended designs to evaluate such initiatives and no guidance on which indicators to use and how to interpret them (Staley 2015). Such methodological developments are greatly needed in order to improve the rigour of patient involvement evaluations. Qualitative and quantitative methodological approaches used to generate patient-based evidence in HTA could also be used to assess the impact of patient involvement from different stakeholder perspectives. For instance, Chap. 9 discusses the notion of PROMs, which could be used to assess the impact of involving patients in HTA on health

outcomes that are meaningful for patients. Qualitative methods, such as ethnographic fieldwork presented in Chap. 12, are particularly relevant to understand the experience of patients and other stakeholders involved in the HTA process.

Qualitative methods are often more appropriate for evaluating an intervention that cannot be isolated from its context. According to Staley (2015), one of the main weaknesses of the current evidence of the value of patient involvement is the lack of detail about the context in which it takes place, thus limiting our understanding of 'why', 'when' and 'how' the involvement has made a difference. The realist evaluation approach adopted by Wilson et al. (2015) offers an interesting avenue to better consider the relation between the context and the mechanisms that lead to outcomes. The GRIPP (Guidance for Reporting Involvement of Patients and Public) checklist (Staniszewska et al. 2011) provides a useful tool to ensure that reporting of results includes a detailed description of where, when, how, why and for whom patient involvement was conducted.

16.5.3 Practical Challenges

With respect to the practical challenges associated with the conduct of patient involvement in HTA, it is important to develop common views on who should be engaged, who they represent, what role they should play, at what stages of the HTA process and using what types of engagement mechanism (Gauvin et al. 2014). Some models have been proposed to guide HTA stakeholders in their patient involvement processes (Gauvin et al. 2014; Gagnon et al. 2015).

Finally, in order to ensure a meaningful contribution of patients in HTA, training opportunities should be offered to them by both HTA bodies and patient associations (Bridges and Jones 2007). The training should include knowledge about HTA in general, the role expected from patients in this exercise and the concepts and terminology used in the specific assessment. There are a few formal training programmes for patient involvement in HTA, for instance, that offered by the HTA programme of the NHS in the United Kingdom (Royle and Oliver 2004), but content of such training should be adapted to the specific context of the HTA and the role that patients have in the process. From our experience, providing training that covers both knowledge on HTA in general and on the specific topic being evaluated, as well as on patient involvement (goal, roles, expectations and process), has been greatly appreciated by patients and other stakeholders involved in a HTA (Dipankui et al. 2015).

16.6 Discussion and Conclusion

While the practice of patient involvement in HTA is developing, rigorous evaluation of its implementation and value is still needed to ensure that it fulfils its objectives. Thus, there must be clearly defined goals for involving patients in HTA such as

those outlined in Chap. 3, which could in turn inform the evaluation questions and the methods selected. It is also important to make sure that patient involvement in HTA is based on a transparent process, that the objectives are clearly defined and that the methods are informed by the best evidence. However, this is not always possible to achieve as HTA producers and patients can understand and practise patient involvement in different ways based on individual ideologies, circumstances and needs. Thus, the simple question of the choice of outcomes as indicators of the success of patient involvement depends on the different perspectives of the key stakeholders (Fudge et al. 2008).

Typical scientific evidence based on experimental designs is currently lacking on the effectiveness of patient involvement interventions on healthcare and health outcomes. As proposed by Drummond et al. (2013), we need to promote the use of rigorous methods for combining and synthesising the findings from qualitative studies. However, alternatives to experimental methods are needed in order to provide rigorous evidence on the added value of involving patients in HTA and to consider factors that contribute to the success of different experiments of patient involvement in HTA (Craig 2008).

Evaluating current experiences is necessary to promote meaningful patient involvement in HTA. Moreover, the question related to the value for money and sustainability of patient involvement initiatives in HTA remains unanswered and would need to be considered if we do not want to 'reinvent the wheel' with every new HTA. Recently, a review of how HTA bodies have evaluated patient involvement in HTA has been undertaken by HTAi, and results are due to be published in 2017. Preliminary findings show that about a half of the HTA bodies that are currently involving patients conduct an evaluation of this involvement.

In this chapter, we have highlighted current practices and issues related to the evaluation of patient involvement in HTA. As the field of patient involvement in HTA is still relatively new, the knowledge base regarding its evaluation and value is likely to expand rapidly. This chapter has also identified the main gaps in knowledge and the challenges associated with the conduct of rigorous evaluations of patient involvement initiatives in HTA. Overall, there are many considerations to take into account when evaluating patient involvement in HTA, and this chapter aimed to provide some practical guidance on how to conduct evaluations that are rigorous and appropriate to each context of patient involvement in HTA.

References

Abelson J, Giacomini M, Lehoux P, Gauvin F-P. Bringing the public into health technology assessment and coverage policy decisions: from principles to practice. Health Policy. 2007;82:37–50.

Abelson J, Montesanti S, Li K, Gauvin FP, Martin E. Effective strategies for interactive public engagement in the development of healthcare policies and programs. Ottawa: Canadian Health Services Research Foundation; 2010. 50 pp.

Boivin A, Lehoux P, Lacombe R, Burgers J, Grol R. Involving patients in setting priorities for healthcare improvement: a cluster randomized trial. Implement Sci. 2014;9:1.

Brett J, Staniszewska S, Mockford C, Herron-Marx S, Hughes J, Tysall C, et al. A systematic review of the impact of patient and public involvement on service users, researchers and communities. The Patient. 2014;7:387–95.
Bridges JF, Jones C. Patient-based health technology assessment: a vision of the future. Int J Technol Assess Health Care. 2007;23:30–5.
Craig GM. Involving users in developing health services. BMJ. 2008;336:286–7.
Dipankui MT, Gagnon MP, Desmartis M, Légaré F, Piron F, Gagnon J, et al. Evaluation of patient involvement in a health technology assessment. Int J Technol Assess Health Care. 2015;31:166–70.
Drummond M, Tarricone R, Torbica A. Assessing the added value of health technologies: reconciling different perspectives. Value Health. 2013;16:S7–13.
Esmail L, Moore E, Rein A. Evaluating patient and stakeholder engagement in research: moving from theory to practice. J Comp Eff Res. 2015;4:133–45.
European Patients' Forum. Patient involvement in health technology assessment in Europe—results of the EPF survey. 2013. http://www.eu-patient.eu/News/News-Archive/Patient-involvement-in-health-technology-assessment-in-Europe—An-interim-report-on-EPF-survey-with-HTA-Agencies. Accessed 28 Oct 2015.
Facey K, Boivin A, Gracia J, Hansen HP, Lo Scalzo A, Mossman J, et al. Patients' perspectives in HTA: a route to robust evidence and fair deliberation. Int J Technol Assess Health Care. 2010;26:334–40.
Fudge N, Wolfe CD, McKevitt C. Assessing the promise of user involvement in health service development: ethnographic study. BMJ. 2008;336:313–7.
Gagnon MP, Desmartis M, Lepage-Savary D, Gagnon J, St-Pierre M, Rhainds M, et al. Introducing patients' and the public's perspectives to health technology assessment: a systematic review of international experiences. Int J Technol Assess Health Care. 2011;27:31–42.
Gagnon MP, Desmartis M, Gagnon J, St-Pierre M, Rhainds M, Coulombe M, et al. Framework for user involvement in health technology assessment at the local level: views of health managers, user representatives, and clinicians. Int J Technol Assess Health Care. 2015;31(1-2):68–77.
Gauvin F-P, Abelson J, Giacomini M, Eyles J, Lavis JN. "It all depends": conceptualizing public involvement in the context of health technology assessment agencies. Soc Sci Med. 2010;70:1518–26.
Gauvin F-P, Abelson J, et al. Evidence Brief: strengthening public and patient engagement in health technology assessment in Ontario. Hamilton: McMaster Health Forum; 2014.
Hailey D, Werkö S, Bakri R, Cameron A, Göhlen B, Myles S, et al. Involvement of consumers in health technology assessment activities by INAHTA agencies. Int J Technol Assess Health Care. 2013;29:79–83.
Ham AJ, Erp N, Broerse JE. Monitoring and evaluation of patient involvement in clinical practice guideline development: lessons from the Multidisciplinary Guideline for Employment and Severe Mental Illness, the Netherlands. Health Expect. 2015;1:471.
Hansen HP, Lee A, van Randwijk CB. Patient aspects: A review of fifty-eight Danish HTA reports. Int J Technol Assess Health Care. 2011;27:330–6.
Hicks N, Hawken NA, Arvin-Berod C, Toumi M. A literature review of patient advocacy group (pag) involvement in HTA. Value Health. 2014;17:A438.
Kellogg Foundation. Using logic models to bring together planning, evaluation, and action: Logic model development guide. Battle Creek: W.K. Kellogg Foundation; 2004.
Kinter ET, Schmeding A, Rudolph I, Bridges JF. Identifying patient-relevant endpoints among individuals with schizophrenia: an application of patient-centered health technology assessment. Int J Technol Assess Health Care. 2009;25:35–41.
Nilsen ES, Myrhaug HT, Johansen M, Oliver S, Oxman AD. Methods of consumer involvement in developing healthcare policy and research, clinical practice guidelines and patient information material. Cochrane Database Syst Rev. 2006;3(3):1.
Oliver SR, Rees RW, Clarke-Jones L, Milne R, Oakley AR, Gabbay J, et al. A multidimensional conceptual framework for analysing public involvement in health services research. Health Expect. 2008;11:72–84.

Rowe G, Frewer LJ. A typology of public engagement mechanisms. Sci Technol Human Values. 2005;30:251–90.

Royle J, Oliver S. Consumer involvement in the health technology assessment program. Int J Technol Assess Health Care. 2004;20:493–7.

Saarni SI, Braunack-Mayer A, Hofmann B, van der Wilt GJ. Different methods for ethical analysis in health technology assessment: an empirical study. Int J Technol Assess Health Care. 2011;27(04):305–12.

Staley K. 'Is it worth doing?' Measuring the impact of patient and public involvement in research. Res Involvement Engagement. 2015;1:1.

Staniszewska S, Brett J, Mockford C, Barber R. The GRIPP checklist: strengthening the quality of patient and public involvement reporting in research. Int J Technol Assess Health Care. 2011;27:391–9.

Van Kammen J, Jansen CW, Bonsel GJ, Kremer JA, Evers JL, Wladimiroff JW. Technology assessment and knowledge brokering: the case of assisted reproduction in The Netherlands. Int J Technol Assess Health Care. 2006;22:302–6.

Wilson P, Mathie E, Keenan J, McNeilly E, Goodman C, Howe A, et al. ReseArch with Patient and Public invOlvement: a RealisT evaluation–the RAPPORT study. Southampton: Health Services and Delivery Research; 2015.

Wortley S, Tong A, Lancsar E, Salkeld G, Howard K. Public preferences for engagement in Health Technology Assessment decision-making: protocol of a mixed methods study. BMC Med Inform Decision Making. 2015;15:52.

Chapter 17
Discussion: Making Sense of Patients' Perspectives, Experiences, and Preferences in HTA

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17.1 The Competitive Advantage of Qualitative Methods

Qualitative research methods have been developed several decades ago and have been applied since then to further knowledge in many social scientific disciplines. Numerous qualitative research communities have been actively sharing their expertise and insights in fields that are closely related to HTA such as sociology of health and illness, healthcare management, health policy, and knowledge synthesis. As a result, there exists today a rich body of scholarship that deepens our understanding of the strengths, limitations, and comparative relevance of specific established qualitative data collection techniques (interviews, focus groups, observation) and explores how innovative qualitative approaches could tap on online environments and tools, including social media (Khodyakov et al. 2016; Marques 2009). Building on this diversified and mature scholarship, four chapters in Part II of this book provide readers with clear guidance on the ways in which particular qualitative methods can help HTA practitioners to elicit patients' perspectives, experiences, and preferences. These chapters also contribute to the science of HTA by making more explicit the epistemological underpinnings of the "patient's view." Along these lines, the current chapter critically discusses the kinds of patient-based evidence one may generate through qualitative methods (1), summarizes key lessons from the four chapters (2), identifies methodological challenges that lie ahead (3), and formulates take-home epistemological messages for the consolidation of patient-based HTA (4).

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17.2 What Kinds of Patient-Based Evidence Qualitative Methods Generate?

Almost two decades ago, Murphy and colleagues produced an important and exhaustive monograph on the role of qualitative methods in the HTA. For these authors, there are some problems in HTA (as introduced in Part I of this book), "which cannot be fully resolved using quantitative methods alone, and there are some circumstances in which qualitative methods represent the technically superior option" (Murphy et al. 1998, p. 87). In our view, what provides qualitative research a "competitive advantage" has to do with its key general features, which include a focus on the perspective of the study participants (be they managers, providers, or patients), an in-depth description of the research setting, a holistic view on the phenomena of interest, an emphasis on processes, and a flexible study design that is responsive to emerging findings.

Typically, qualitative research is suited to address "why" and "how" research questions, and it does so by recognizing the centrality of subjectivity in human action: "Health technologies are applied by people (be they doctors, nurses, technicians or patients) to other people (usually patients). One of the distinctive features of a human action is that it is meaningful. People act on the basis of what they believe to be true rather than what may be objectively true" (Murphy et al. 1998, p. 87).

Because their object of inquiry is permeated by subjectivity, qualitative researchers have developed theoretical frameworks and methodological tools in order to handle rigorously their informants' subjectivity as well as their own subjectivity (better than their quantitative colleagues, we would be tempted to say). Such scholarly advancements have relied not exclusively but often on constructivist epistemologies, which problematize the relationship between the "knower" and the knowledge being produced (i.e., reflexivity), and on social scientific theories, which are necessary to make sense of meanings and social interactions.

Together, epistemological and theoretical frameworks define what count as knowledge, how such knowledge can be produced, what is knowable, and why it should be known. Because "the choice of theory, although often unacknowledged, shapes the way practitioners and researchers collect and interpret evidence" (Alderson 1998, p. 1007), qualitative research that is not firmly grounded in a social scientific framework suffers from severe limitations (perhaps like a quantitative study of poor quality that has insufficient power to detect a statistically significant change).

For Murphy et al. (1998, p. 87), qualitative research brings an important contribution to HTA "whenever the context in which a health technology is to be implemented can be expected to have an impact upon the outcome of that technology." Qualitative studies can clarify the organizational, political, and sociocultural dimensions that affect the diffusion of technology in different settings as well as its real-world effectiveness. According to the Canadian Health Services Research Foundation, three categories of evidence bring a distinct contribution to health

policy: (1) context-free scientific evidence (such as the knowledge generated through a randomized controlled trial on the efficacy and safety of a new technology), (2) context-sensitive scientific evidence (such as the knowledge generated by an implementation study that examines regional variations using a sociology of innovation framework), or (3) colloquial evidence, which refers to the expertise, views, and realities of stakeholders (CHSRF 2006, p. 5). The term "colloquial evidence" was hotly debated in the Canadian health services and policy research community; the idea behind the Foundation's initiative was to recognize a form of knowledge that often remains informal, but which is very valuable in health policy-making since it sheds light on "resources, expert and professional opinion, political judgment, values, habits and traditions, lobbyists and pressure groups, and the particular pragmatics and contingencies of the situation" (CHSRF 2006, p. 1). By drawing on these three categories of evidence, it becomes possible to develop a more patient-centered HTA.

17.3 How to Generate Qualitative Evidence for HTA: Key Lessons from the Four Chapters

Each of the four chapters (12, 13, 14, and 15) focuses on a particular qualitative method or approach to elicit and synthesize patients' perspectives, experiences, and preferences. While Street and Farrell address the emerging and still indeterminate potential of social media, the other chapters bring to the readers' attention methodologies for which there is now much more scholarship available. Below, we address successively each chapter's key contributions and critical lessons for the international HTA community to ponder.

17.3.1 Ethnography Is More than the Sum of "n" Individual Interviews, and Focus Groups are not a Cheaper and Quicker Means to Increase One's "n"

For Tjørnhøj-Thomsen and Hansen (Chap. 12), ethnography is first and foremost concerned about the patient's everyday life and context, not just about their discourse regarding a particular service, technology, or policy. Because ethnography pays attention to the social situation in which the use of a technology unfolds (defined by the place, actors, and activities involved), it leads to a detailed understanding of how particular settings influence its real-world effectiveness. Hence, ethnography goes well beyond the knowledge one may obtain from conducting a series of "x" interviews since it is designed to capture holistically the interactions between the technology, patients, and their social surroundings, including providers, insurers, employers, neighbors, family, etc.

These authors also aptly underscore that the nature of the technology—that is, whether its use is lifesaving or not, whether it supports chronic or acute care, or whether it involves small or large trade-offs in one's personal life—will influence the relevance of using a particular method to gather patients' perspectives. The tacit desire to develop a "one-size-fits-all" approach to generate patient-centered evidence appears unsound and would divert the attention from a key object of inquiry in HTA: technology's impact on patients.

While it is true that ethnography is necessarily time-consuming, one of its particular strengths lies in its long duration. Considering that some patients may be "of a few words" but certainly not without intent, expectations, or emotions, the time ethnographers patiently invest in the field is directly proportional to the completeness of the analyses they will be able to achieve. Just like focus groups are not a cheaper and quicker means to increase one's "n," we concur with Tjørnhøj-Thomsen and Ploug Hansen when they stress that ethnography is not a simple exercise of collecting or gathering patients' views, but a research endeavor per se. When critically examining focus group research that we had conducted with patients, we argued that such a method does not "derive epistemological authority simply because of the identity of its participants" (Lehoux et al. 2006, p. 2103). Researchers need to recognize that patients do not arrive "with a logically coherent system of pre-formed ideas that just need to be skillfully elicited or discovered. Nor do they share in any straightforward way their knowledge or naively endorse all knowledge claims put forward by others" (Lehoux et al. 2006, p. 2103).

This is one of the reasons why ethnography must be understood and practiced as a comprehensive research endeavor. Tjørnhøj-Thomsen and Hansen rightly tear to pieces the assumption according to which qualitative research would not require special training and expertise. A rigorous, in-depth understanding of what works and does not work in particular settings is very precious when policymakers and practitioners are looking for ways to adequately implement new technologies that raise similar patient-related challenges. This is why, despite the time it takes, ethnographic research is likely to provide results with a profound and lasting usefulness, thereby supporting the broader mission of HTA.

17.3.2 Deliberation Aims to Produce More than a Collection of Opinions, and, as Such, It Constitutes a Demanding Process for Patients and Researchers

Street and Lopes (Chap. 13) provide readers with a comprehensive introduction to the use of deliberative methods in patient-centered HTA, from their democratic theoretical aspirations to their ethical and methodological specificities. Deliberation brings the elicitation of patient's perspectives, preferences, and experiences to another analytical level: what matters is not to collect a range of views, but to bring these views in a collective dialogue (Bombard et al. 2011; Degeling et al. 2015). The goal of deliberation is to reflect on and ponder what may seem like a reasonable

collective course of action. This is why Street and Lopes carefully define the principles underlying a "good deliberation" as well as the trade-offs that are associated to various deliberative democracy models.

While a deliberative intervention in HTA may be organized around either a policy or research question, it has to explicitly support the expression of challenging viewpoints and learning opportunities for all participants (Abelson et al. 2010; de Vries et al. 2011). As Giacomini and Cook underscore (2000, p. 480), dialogue "affects the meanings of social experiences, and the results of a dialogue translate these experiences for persons who might not otherwise understand each other's perspectives well." Beyond the necessary translation between different perspectives, a dialogue may also prove transformative. A puzzling issue when one seeks to design (and later assess) a deliberative intervention is to clarify what kind of transformation one expects exactly (Carman et al. 2014). For some scholars, participants have to be selected by ascertaining whether they can prove flexible in their thinking or not to hold "too strong views." This would entail applying certain exclusion criteria, which is obviously an intricate issue that may undermine the legitimacy of the whole deliberative endeavor. Moreover, it might be entirely legitimate that some opinions remain unchanged if, at the same time, mutual learning between participants takes place (Black et al. 2011; Lehoux et al. 2009).

More specifically, Street and Lopes describe five dimensions that may be seen as key ingredients to a deliberation of quality (Table 14.1). They also aptly underscore the need to reach out to "unsuccessful" patients, that is, patients for whom new promising treatments may have failed. Yet, these authors bring to the readers' attention how deliberative methods may prove physically and/or emotionally burdensome, and this, in itself, creates a formidable tension when the aim is to gather the views of individuals who are already afflicted by health problems.

While deliberative methods are increasingly being applied in the health field, their evaluation "continues to be carried out in the absence of any guiding frameworks that define the theoretical basis for the public engagement process or the relationships among the public engagement mechanism and process or outcome variables of interest" (Abelson et al. 2010, p. 10). For Popay (2014) and the Public Involvement Impact Assessment Framework (PiiAF) Study Group, the "intervention theory" should be made explicit by providing a description of the ways in which a particular approach to involving patients will lead to the expected effects. Such recommendations are likely to further the informed development of deliberative methods in HTA.

17.3.3 Social Media May Be at Risk of Remaining an Unrealized Opportunity If One Does Not Recognize the Need to Apply Mixed Methods

Street and Farrell (Chap. 14) bring a much-needed contribution by addressing the fast, moving domain of social media-based research, which opens up an array of "(as yet largely) unrealized opportunities." Among the key arguments for exploring

how social media may be used to elicit patients' perspectives, experiences, and preferences in HTA, one finds the barriers that limit the participation of specific individuals and groups to traditional, face-to-face qualitative data collection methods. Such barriers may be physical, geographical, sociocultural, or a mixture of these. For instance, there are many topics in HTA that raise social desirability or sensitive issues (e.g., sexual health, substance abuse) that require recruiting over a very large area (e.g., rare diseases, discriminatory practices based on gender, handicap, or ethnicity) or that call for the ability to secure collaboration with hard-to-reach groups (e.g., migrants, stigmatized lifestyles). For many observers, and not unlike the hype that surrounded telemedicine in the mid 1990s, social media would easily reduce if not eliminate all of these barriers.

While we clearly share the enthusiasm of Street and Farrell toward the use of online environments in research, a number of contentious methodological issues need to be tackled before social media-based research may deliver its promises. Right at the outset, one has to define more precisely what social media-based research is and what it is not. For instance, social media differ from online surveys and online interviews, which respectively enable the gathering of quantitative and qualitative data. The "quasi" qualitative nature of the data social media may provide access to needs to be acknowledged. For instance, if one may create a "snapshot of views" by aggregating a large number of online posts, such research may fall short of fulfilling the essential qualitative research features we introduced earlier. It would amount to a quantification of qualitative data that may not provide context-sensitive in-depth interpretations.

As Street and Farrell aptly stress, online environments are fluid, they change rapidly, they lack social cues and nuances, and knowing who is talking exactly (for instance, health technology industry-sponsored and/or physician-led patient groups) remains at times an act of faith. One puzzling issue researchers face is to define what a purposeful (or reasoned) "sample" may be when geographical boundaries are made more or less irrelevant and when online identities are made explicitly plural by those using social media to share their views and experiences.

Hence, we would be careful before recommending that "disinvestment policy scenarios" rely on such methods and have doubts that using "recognized" patient associations may increase legitimacy in the eyes of HTA stakeholders. In our view, HTA scholars and practitioners may even have to slow down policymakers' demand for online tools, especially "in situations where gathering people in an in-person venue is difficult or impractical" (Carman et al. 2014, p. 109). One cannot underestimate current digital divides around the globe and within individual countries as well as the inequalities varying levels of e-health literacy may reinforce. In our view, the risk is that online tools be "used as a standalone, second-best method, which may increase civic inequalities in countries with a geographically dispersed population" (Lehoux et al. 2016, p. 13, Marques 2009).

Because of the volume of data available and their discursive and (self-) representational nature, social media-based research may, to a certain extent, be compared to media coverage analysis. While one may analyze what is said on social media, it remains difficult to clarify why it is said and with what impact on social media users. This is why we believe that it may prove more realistic and productive to

apply to social media-based research an integrated mixed method approach that would cohesively combine detailed qualitative interpretations and meaningful quantitative measures. Overall, we disagree with the idea that social media could be used "to conduct relatively fast, inexpensive and feasible" qualitative research. Yet, the methodological challenges raised by social media-based research are likely to be reduced as more scholars study how patients mobilize and make sense of social media throughout their illness trajectories.

17.3.4 Doing Without Qualitative Evidence Synthesis Is Not Anymore an Option

In Chap. 15 by Booth, one finds an enlightening and state-of-the-art description of the reasons why qualitative evidence synthesis has become an inclusive term and an important tool in HTA. Key distinctions between such syntheses and traditional systematic reviews include their "configurative rather than aggregative" nature, which implies drawing meta-theoretical links between different types of empirical findings, and their bibliographic search strategies, which require a strong command of the specificities of biomedical and social scientific publication databases alike.

While there are "few worked examples" of qualitative evidence synthesis, an increasing number of meta-ethnographies are published every year. This can be understood as a result of the sheer number of publications that are already "out there"—and hard to ignore—and of the natural connection between qualitative research and the "patient's view." In other words, HTA practitioners can no longer begin a new study without taking stock of the available published qualitative evidence on the topic. The good news is that synthesis methodologies have made an enormous leap forward in the past decade, exemplified by the international efforts Booth describes and which have produced and shared tools and methodological resources. Such efforts are particularly important since rigor in qualitative evidence synthesis cannot depend upon a hierarchy in study designs.

Interestingly, while each individual published qualitative study may never have been "intended to be generalizable," a solid synthesis methodology may contribute to increase their scope and policy impact. When reviewing a set of qualitative studies that address a similar topic, it becomes possible to identify divergent and convergent findings across and within different populations and settings. A qualitative evidence synthesis, thanks to its "interpretative richness," can help to piece together why and how such variations occur. In the quest to support patient-focused HTA, one piece of the methodological puzzle that Booth clarifies very well is the distinction between a synthesis of the patients' experience of a condition and a synthesis of their appreciation of the outcomes of a particular technology or service. Whereas the former will necessarily be broader in scope and require some theory building (i.e., configurative), the latter may prove much more focused and potentially less time-consuming (i.e., aggregative). Henceforth, for an HTA body not to engage in the production of qualitative evidence syntheses would reveal an ideological rather a methodological decision.

17.4 What Do Future Methodological Challenges Lie Ahead?

Considering the time and resources constraints that typically plague HTA bodies, one may wonder whether these HTA bodies are able to conduct the kind of qualitative primary research described in Part II of this book. Engaging in the production of qualitative evidence syntheses seems more realistic. Yet, for an HTA body to be able to tap on the competitive advantage of qualitative methods, it has to hire or subcontract researchers who possess such research skills and experience. HTA producers who are generally trained to perform quantitative systematic reviews would have to learn how to read critically qualitative studies and be able to meaningfully extract from social scientific studies evidence that is relevant to patients' perspectives, preferences, and experiences. They may also have to develop methodological skills in the design of syntheses that integrate both kinds of evidence as suggested by Booth.

When it is not feasible to conduct a synthesis of qualitative evidence, for instance, in the case of emerging technologies, performing qualitative primary research may prove necessary. Preliminary ethnographic fieldwork or other qualitative methods could be indicated in those circumstances. It would provide at the same time a great opportunity to engage patients in the research process itself.

Among the future methodological challenges that the HTA qualitative research community will have to handle is the place face-to-face data collection methods should occupy within a digital world. For instance, deliberative interventions that rely on audiovisual material-based tools may succeed in supporting informed online deliberations among nonexperts (Lehoux et al. 2016; Lehoux et al. 2014). Nonetheless, such online approaches may have to be combined with face-to-face methods in order to foster inclusiveness and meaningful involvement of all participants, thereby maximizing the opportunity to democratically learn from each other and co-produce rigorous patient-centered knowledge (Khodyakov et al. 2016).

17.4.1 Take-Home Epistemological Messages

Beyond knowing how to choose and apply the right methods, one needs to unpack the epistemological underpinnings of patient-based evidence. As Tjørnhøj-Thomsen and Hansen point out, while patient preferences and patient experiences both reflect patients' perspectives, they are two different objects of inquiry. Moreover, patients' perspectives on these objects are "emerging, relational, and shifting." This observation has tremendous implications since it brings us back to the task of defining what counts as patient-related knowledge, how such knowledge can be produced, what is knowable, and why it should be known. While Booth underscores that a constructivist orientation in qualitative methods may prove less directly relevant to decision-makers, we believe that it prevents from "positing an apparent consensus as *the*

patient's view" since it explicitly recognizes how such views remain "the result of context-dependent social interactions wherein perplexing dynamics contribute to the creation of dominant narratives" (Lehoux et al. 2006, p. 2103).

As underscored by Street and Lopes, methods to elicit patient's perspectives, experiences, and preferences are likely to deliver the diversity a deliberative democracy requires. Yet, one then needs to clarify the policy implications of such diversity. Here it is not so much the type of evidence being gathered that is at play, but the relationship HTA entertains with the policy sphere. The credibility and legitimacy of an HTA body that ventures into the generation of patient-based evidence will be increased if it can consistently show a strong command of qualitative methods and, as underscored by Tjørnhøj-Thomsen and Hansen, this is not a mere *technical* challenge. Rigor in qualitative research requires a strong ability to *think qualitatively* and therefore to reflexively address the participants' and one's own subjectivity.

17.4.2 Concluding Remarks

In this chapter, we argued that qualitative research methods cannot reasonably be considered an "emerging" topic anymore by HTA producers. The qualitative research scholarship is vast, mature, and dynamically responsive to the digital world in which our societies evolve. Furthermore, when it comes to making sense of patients' perspectives, experiences, and preferences, it possesses a clear competitive advantage over quantitative methods since it can clarify why, how, and in what context patient-based evidence-based healthcare may flourish.

Yet, like any other specialized scientific endeavor, qualitative methods call for a specific body of knowledge, know-how, and skills. Such methodological expertise has to be rigorously acquired and applied. It often also requires a social scientific "lens"; otherwise, one may be trying to piece together different data fragments without using an explicit, consistent theoretical framework.

We do not believe that there is neither a "quick and fast" way to become a solid qualitative researcher nor a "simple and cheap" way to produce rigorous and non-complacent patient-based evidence. HTA scholars and practitioners should therefore resist ill-informed policy demands for such kind of diluted evidence since they entail a waste of precious human and financial resources. Providing policymakers with instant coffee may temporarily relieve them from a lack of caffeine, but, in the long run, they are likely to miss the very substance that makes a coffee a coffee, including the long and taxing process by which the coffee beans are grown and harvested, packaged and shipped, and roasted and ultimately brewed.

In other words, HTA as a field must stay true to the rich, complex, and at times conflicting realities of patients. Fulfilling this aspiration requires, beyond the necessary allocation of time and resources, a reflexive, theory-informed, and rigorous distillation of large amounts of qualitative data, which will improve our ability to account for technology's role in patients' everyday life and context.

References

Abelson J, Montesanti S, Li K, Gauvin F-P, Martin E. Effective strategies for interactive public engagement in the development of healthcare policies and programs. Ottawa: Canadian Health Services Research Foundation; 2010. 49 pp.

Alderson P. The importance of theories in health care. BMJ. 1998;317:1007–10.

Black LW, Burkhalter S, Gastil J, Stromer-Galley J. Methods for analyzing and measuring group deliberation. In: Bucy EP, Holbert RL, editors. Sourcebook of political communication research: methods, measures, and analytical techniques. New York: Taylor & Francis; 2011. p. 323–45.

Bombard Y, Abelson J, Simeonov D, Gauvin FP. Eliciting ethical and social values in health technology assessment: a participatory approach. Soc Sci Med. 2011;73:135–44.

Carman KL, Maurer M, Mallery C, Wang G, Garfinkel S, Richmond J et al. Community forum deliberative methods demonstration: evaluating effectiveness and eliciting public views on use of evidence. Final report Nov 2014. Washington: Agency for Healthcare Research and Quality. 167 pp. AHRQ Publication No. 14(15)-EHC007-EF. American Institutes for Research under Contract No. 290-2010-00005.

CHSRF, Canadian Health Services Research Foundation. Weighing up the evidence. Making evidence-informed guidance accurate, achievable, and acceptable. A summary of the workshop held on September 29, 2005. 2006. http://www.cfhi-fcass.ca/migrated/pdf/weighing_up_the_evidence_e.pdf Accessed 15 Oct 2016.

De Vries R, Stanczyk AE, Ryan KA, Kim SY. A framework for assessing the quality of democratic deliberation: enhancing deliberation as a tool for bioethics. J Empir Res Hum Res Ethics. 2011;6:3–17.

Degeling C, Carter SM, Rychetnik L. Which public and why deliberate? A scoping review of public deliberation in public health and health policy research. Soc Sci Med. 2015;131:114–21.

Giacomini MK, Cook DJ. Users' guides to the medical literature: XXIII. Qualitative research in health care B. What are the results and how do they help me care for my patients? Evidence-Based Medicine Working Group. JAMA. 2000;284:478–82.

Khodyakov D, Savitsky TD, Dalal S. Collaborative learning framework for online stakeholder engagement. Health Expect. 2016;19:868–82.

Lehoux P, Poland B, Daudelin G. Focus group research and "the patient's view". Soc Sci Med. 2006;63:2091–104.

Lehoux P, Daudelin G, Demers-Payette O, Boivin A. Fostering deliberations about health innovations : what do we want to know from the publics? Soc Sci Med. 2009;68:2002–9.

Lehoux P, Gauthier P, Williams-Jones B, Miller FA, Fishman JR, Hivon M, et al. Examining the ethical and social issues of health technology design through the public appraisal of prospective scenarios: a study protocol describing a multimedia-based deliberative method. Implement Sci. 2014;9:81.

Lehoux P, Jimenez-Pernett J, Miller FA, Williams-Jones B. Assessment of a multimedia-based prospective method to support public deliberations on health technology design: participant survey findings and qualitative insights. BMC Health Serv Res. 2016;16:616.

Marques ACS. La conversation civique Sur internet: contributions au processus délibératif. Estudos em Comunicação/Commun Stud. 2009;5:21–52.

Murphy E, Dingwall R, Greatbatch D, Parker S, Watson P. Qualitative research methods in health technology assessment: a review of the literature. Health Technol Assess. 1998;2:3–9. 1-274.

Popay J, Collins M, (editors) with the PiiAF Study Group. The public involvement impact assessment framework guidance. Universities of Lancaster, Liverpool and Exeter; 2014. 69 pp.

Chapter 18
Discussion: Research to Promote Patient-Based HTA

John F.P. Bridges and Ellen M. Janssen

18.1 Introduction

This chapter provides an overview of research methods that can be used to enhance patient collaboration and incorporate patients' perspectives into HTA. The first part of this chapter discusses the foundations of patient-based HTA. First, we discuss two different approaches to patient-based HTA, the collaborative approach and the scientific approach, and overview the ethical and practical implications of patient collaboration in HTA. Secondly, we give a brief summary of the chapters covering patient-reported outcome measures (PROMs), discrete choice experiments (DCEs), and analytical hierarchy processes (AHPs) as examples of the scientific approach to patient-based HTA. In the second part of this chapter, we discuss next steps and gaps surrounding methods that can be used in patient-based HTA. It is clear that patient-based HTA has regained popularity in recent years, yet more research is needed to explore the validity and generalizability of methods that measure patients' perspectives and how to incorporate them into patient-based HTA. We caution that more care needs to be taken to ensure that collaboration with patients and patient groups is based on mutual respect and that patients' perspectives are clearly represented. We conclude the chapter by discussing what is currently missing in patient-based HTA and call for comprehensive guidance on patient-based HTA with increased transparency and collaboration across researchers, patients, agencies, and other stakeholders that want to advance patient-based HTA.

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18.2 Foundations

The original goal of HTA was to be an organized, transdisciplinary effort to assess the intended and unintended consequences of new medical technologies (Chap. 1). While there are many who still want to live up to these lofty goals (Banta 2003), one could easily be mistaken that HTA has been hijacked by health economists who are focused only on the payers' perspective of medicine (Bridges 2005; Henshall et al. 1997). It was from the shadows of this "cost-per-QALY" paradigm, most dominant in the first decade of this millennium, that an alternative paradigm that placed the patient at the heart of HTA regained strength (Bridges 2006a). To be more correct, it would be appropriate to think of these patient-centered efforts like a resistance movement. Like many other empowerment movements in society, the concept of patient centeredness is best described in its absence (Vogt et al. 2006). Initially this counterrevolution was fragmented—a series of isolated efforts targeted at chipping away at the dominance of cost-per-QALY thinking (Bridges and Jones 2007). Over time, however, it has become more organized, more consolidated, more scientific, and eventually, more accepted in HTA (and more broadly in medicine) (Abelson et al. 2007). In this chapter, we summarize some of the main factions that have emerged in this movement to patient-based HTA, many of which have been discussed in the previous chapters; we ask if these efforts have been impactful and speculate as to what lays ahead for patient-focused HTA.

While patient-based HTA emerged circa 2004–2006, there is no denying that its foundations can be found in earlier grassroots disease and patient advocacy efforts (Bastian 2000). Identifying the exact antecedents of this crossover into HTA is hard to determine. Was it an endogenous "push" effort on behalf of these advocates who were seeking a seat at the table, or were these advocates actively pulled into HTA by those who saw the injustices of a payer-centered cost-per-QALY exercise? Alternatively, one can see these first efforts to create patient-centered outcomes research as a purely theoretical exercise—a dream of an alternative evaluation paradigm that was either more patient centered (Loukanova et al. 2007) or one that had a stronger theoretical grounding than the prevailing paradigm of cost-effectiveness analysis (Bridges 2003; Bridges 2006b; Rotter et al. 2012).

18.3 Two Schools of Thought

Irrespective of the foundations of the movement, there were two broad schools of thought that emerged in these early years – and these have tended to prevail over the years. The first was focused on supporting patients to participate through representation, consultations, and testimony (Chap. 6). Different to qualitative research, as it was often neither a systematic nor generalizable research effort, this participation movement was aimed at getting patients' voices into the same room as the decision-makers or to enlist patient representatives in active decision-making.

The second school of thought of incorporating patients' voices into HTA was focused on the use of scientific methods to document the needs, experiences, and preferences of patients and other stakeholders to explicitly inform HTA and other medical decisions (Chaps. 9, 10, 11). This second movement focused on research and was predominantly driven by university researchers from diverse disciplines. These academics were quickly joined by a broader cadre of researchers from consulting and the pharmaceutical industry who saw an opportunity to use patient-focused research as a mechanism to broaden the criteria used to judge innovation and value (Bridges et al. 2008).

This second school of thought could be simply dichotomized into a qualitative and quantitative approach that focused on either telling a rich/complex narrative (Hansen et al. 2011) or a parsimonious/numeric approach (Donaldson 2001), but–as the chapters above indicate–such a dichotomy would be flawed. Furthermore, despite the obvious need and numerous attempts to develop a better taxonomy to distinguish these efforts (Coast et al. 2012, MDIC 2015[1]), there has been very little progress in conceptualizing the various research methods (Chap. 4). The residual confusion that persists as to how these methods are classified has certainly acted as a barrier to the broader dissemination and implementation of patient-focused scientific efforts in HTA and in medicine more generally.

18.4 Patients Collaborating in HTA Research and Evaluation

To a large extent, Chaps. 8 and 16 deal with the subject matter consistent with the first school of thought–patients collaborating in HTA—with de Wit and Gossec focusing on patients' participation in clinical research and Gagnon and colleagues focusing on evaluating the impact of involving patients in HTA. This is not to say that the topics covered in the other chapters in Sect. II have ignored the concept of patient collaboration. For example, multi-criteria decision analysis (MCDA) methods have now become more common in HTA circles, and techniques like analytic hierarchy process—covered in Chap. 11 by Danner and Gerber-Grote—could be utilized in getting patients and other stakeholders more actively involved in the deliberative process(es) within HTA (Hummel et al. 2014).

The lack of relevant case studies in HTA emphasizes that patient collaboration in HTA is an evolving field in which stakeholders should adopt an attitude of co-learning and maintain flexibility in their partnership approaches as the field evolves. While, from an ethical standpoint, patient involvement should be sought regardless of its "added value," evaluating patient involvement can help stakeholders learn from the work of others and can result in the improvement of collaborative practices. Thorough evaluation of patient involvement in HTA could help guide patient

[1] Medical Device Innovation Consortium.

involvement; it could also result in practices that meet evaluation guidelines but are not in the spirit of true collaboration. Therefore, especially in the early stages of patient-based HTA, funding agencies and other stakeholders will need to provide flexibility to allow for true patient-researcher collaboration.

18.5 Scientific Research Methods

While the use of scientific research methods to capture the perspectives of patients and other stakeholders relevant to HTA are not necessarily superior to efforts that support patients to participate in HTA, they do serve important roles in promoting patient-based HTA. Scientific methods allow researchers to capture the perspectives of a larger group of patients than those that are able to directly participate in HTA processes. Most importantly, the use of science has been justified in combating the perspective that the patients' perspective is the "soft side" of HTA.

Chapters 9–15 of this part deal with various research methods to capture patients' perspectives. Given that those chapters that deal more with qualitative methods have been eloquently discussed by Lehoux and Jimenez-Pernett, this section will focus on the methods that are more quantitative in nature. Again, the qualitative/quantitative dichotomy may be a suboptimal way to categorize scientific research methods targeted at incorporating patients' perspectives into HTA. This said, this dichotomy between qualitative and quantitative methods has been categorized elsewhere (Curry et al. 2009).

Chapters 9, 10, and 11 of this volume detail three emerging factions targeted at quantifying patients' perspectives. The chapter by Haywood and colleagues details techniques for developing robust and relevant PROMs for use in HTA. Tockhorn-Heidenreich and colleagues discuss the use of DCEs (a common example of a broader class of stated-preference methods) in quantifying preferences of patients and other stakeholders. Finally, Danner and Gerber-Grote discuss the use of AHP (a common example of MCDA methods) in eliciting patient preferences. While this is not an exhaustive set of quantitative methods aimed at capturing the perspectives of patients and other stakeholders, they do represent three of the most influential factions within this movement.

18.5.1 Measuring What Matters to Patients

The PROM has quickly gained in prominence in the use of clinical trials and healthcare decision-making with the intent to bridge the gap between clinicians' and healthcare decision-makers' understanding of the effect of disease and the patient experience. PROMs might capture symptom status, physical function, mental health, social function, and well-being (Nelson et al. 2015). As Haywood and colleagues point out in Chap. 9, well-developed PROMs reflect patients' perspectives

and can therefore help HTA bodies understand the true value of a health technology. However, researchers or third party stakeholders have generally pushed the PROM movement forward with limited consideration of patients' perspectives. Therefore, many PROMs are not patient centered or patient relevant (Wiering et al. 2016; Trujols et al. 2013). Patients should be involved in the development of the PROM from item identification to refinement and evaluation (Staniszewska et al. 2012). If PROMs succeed in capturing some of the patient experience, they have tremendous potential to move patient-based HTA away from simple cost-per-QALY thinking, especially if patient-motivated importance weights can be assigned to different outcomes.

18.5.2 Stated-Preference Methods

In Chap. 10, Tockhorn-Heidenreich and colleagues discuss four steps to conducting a DCE, a type of stated-preference method, and discuss methodological challenges of this method within an HTA context. DCEs provide a robust set of tools for quantifying people's priorities, preferences, and values and have been applied in market research, transportation, environmental policy, and health (Hauber et al. 2013). Guidelines on conducting DCEs have emerged in recent years (Bridges et al. 2011; Johnson et al. 2013; Hauber et al. 2016). However, since DCEs are conducted in tightly controlled experimental settings and ask participants to make hypothetical choices, it is not clear whether participants would display the same preferences if they experienced the consequences of their choices. Checks on the internal logic and consistency of DCE studies, how participants make choices, and for the external validity and generalizability of DCEs are needed. Furthermore, techniques for developing DCEs and for incorporating DCE results into HTA and other decision-making processes need to be examined.

18.5.3 Multi-Criteria Decision Analysis Approaches

Danner and Gerber-Grote discuss AHP, a form of MCDA in Chap. 11. AHP can be used to structure and support complex decision problems in healthcare (Hummel et al. 2014). AHP can serve to increase the transparency of decision-making processes and to elicit preferences for decision criteria. These methods raise a number of questions regarding preference elicitation that have not been answered yet. When preferences vary between patients, clinicians, or other stakeholders, decision-making processes need to account for these different preferences. As more research is done on AHP and MCDA, it remains important to be transparent about the limitations of these methods (and other methods as well), especially since one of the objectives of patient-based HTA is to create a more transparent decision-making process.

18.6 What is Next?

As patient-based HTA is starting to become more common, it is important to ensure that researchers do not view patient collaboration as simply another box to check. This attitude could lead to token involvement and undermines the philosophy of co-learning and mutual respect that should be the basis of patient-researcher collaboration. Existing frameworks on how patients can and should be involved often maintain a paternalistic and protective approach that directly contradicts the spirit of equal partnership. Therefore, to truly reflect an environment of collaboration between patients and other stakeholders, patients should be involved as experts on living with the condition of interest.

While guidelines on designing PROMs and conducting DCE and AHP studies exist, questions regarding the application of these studies remain. First, researchers will need to conduct studies to examine the validity and generalizability of patient-centered results and will need to develop tests that can be used to evaluate the validity of a patient-centered studies. In doing this, the different scientific methods should learn from each other; for example, MCDA researchers might be able to adapt some, but not all, of the validation approaches currently used for PROMs. In addition, further research comparing preference methods might shed light onto the quality and applicability of different approaches. For example, many researchers prefer DCE over AHP because DCE is grounded in economic theory (random utility theory (McFadden 1974)), while AHP is not. More studies that compare the performance of these methods might provide insight into the merit of these concerns (Ijzerman et al. 2012). Secondly, frameworks on how to incorporate patient research partners in PROMs, DCEs, and AHP should be developed to aid researchers new to patient involvement. Thirdly, ways to combine the results of the different patient-centered methods should be incorporated. For example, conducting a DCE on PROM items could help assign importance weights to each of the items in the PRO instrument.

18.6.1 What is Missing?

Patient partnership collaboration in HTA is an evolving field in which researchers, decision-makers, and other stakeholders should be encouraged to share experiences and adjust approaches as the field evolves. Guidance on design and how to conduct particular scientific studies to elicit patient preference information, such as DCE, have been developed. However, a set of good research practices or guidance documents on patient involvement and collaboration could help those that are new to patient-based HTA to effectively implement patient partnerships. These documents would need to emphasize the evolving nature of patient-based HTA. In addition, to encourage patient involvement, funding agencies and other stakeholders might need to become more aware of the challenges that patient collaboration can bring and put in place flexible mechanisms to address these challenges.

A connected network of researchers, patient advocates, and other stakeholders actively involved in patient-centered research and decision-making could help accelerate patient involvement in healthcare. For example, a comprehensive taxonomy of patient-based efforts in HTA and other decision-making mechanisms would allow stakeholders to learn from each other's initiatives. Section III of this book provides an overview of patient-based efforts around the world, but this list will need to be continuously updated to reflect the ever growing and changing field of patient involvement in healthcare decision-making.

18.7 Conclusion

In this chapter, we discuss the rise of patient-based HTA as a counter movement to traditional cost-effectiveness approaches. We discuss the foundations of patient-based HTA within the context of disease and patient advocacy and examine the emergence of two schools of thought within patient-based HTA: the representation school of thought and the scientific methods school of thought. We give a brief overview of the first school of thought by discussing Chap. 8 in which de Wit and Gossec examine patient collaboration in HTA and Chap. 16 in which Gagnon and colleagues discuss the need to evaluate patient involvement in HTA. We then summarize the scientific methods school of thought by briefly discussing material covered in Chaps. 9, 10, and 11 on PROMs, stated-preference methods, and MCDA.

As patient-based HTA evolves, it is clear that these two schools of thought do not need to be separate. Patients as collaborative research partners can aid in the design and interpretation of scientific studies that are meant to capture their perspectives. Furthermore, the presence of one or more patient representatives on HTA boards does not preclude the use of patient-centered evidence obtained from PROMs, DCEs, or AHP. As patient-based HTA evolves, patients will increasingly need to be involved as equal partners on research and decision-making boards. Patients can help interpret evidence on the perspectives of larger groups of patients. To ensure the adoption of patient-based HTA, clear but flexible guidelines on the use of patient-centered methods should be established, and properties of validity and reliability of patient-centered studies need to be examined.

References

Abelson J, Giacomini M, Lehoux P. Bringing 'the public' into health technology assessment and coverage policy decisions: from principles to practice. Health Policy. 2007;82:37–50.

Banta D. The development of health technology assessment. Health Policy. 2003;63:121–32.

Bastian H. Allies or enemies? Evidence-based medicine and consumer choice. Med J Aust. 2000;172(1):5–6.

Bridges J. Stated preference methods in health care evaluation: an emerging methodological paradigm in health economics. Appl Health Econ Health Policy. 2003;2:213–24.

Bridges J. Future challenges for the economic evaluation of healthcare: patient preferences, risk attitudes and beyond. PharmacoEconomics. 2005;23:317–21.
Bridges J. What can economics add to health technology assessment? Please not another cost-effectiveness analysis! Expert Rev Pharmacoecon Outcomes Res. 2006a;6:19–24.
Bridges J. Lean systems approaches to health technology assessment: a patient focused alternative to cost-effectiveness analysis. PharmacoEconomics. 2006b;24(Suppl. 2):101–9.
Bridges J, Jones C. Patient based health technology assessment: a vision of the future. Int J Technol Assess Health Care. 2007;23:30–5.
Bridges J, Kinter E, Kidane L, Heinzen RR, McCormick C. Things are looking up since we started listening to patients: recent trends in the application of conjoint analysis in health 1970–2007. Patient. 2008;1:273–82.
Bridges J, Hauber AB, Marshall D, Lloyd A, Prosser LA, Regier DA, et al. Conjoint analysis applications in health—a checklist: a report of the ISPOR good research practices for conjoint analysis task force. Value Health. 2011;14:403–13.
Coast J, Al-Janabi H, Sutton EJ, Horrocks SA, Vosper AJ, Swancutt DR, et al. Using qualitative methods for attribute development for discrete choice experiments: issues and recommendations. Health Econ. 2012;21:730–41. doi:10.1002/hec.1739.
Curry LA, Nembhard IM, Bradley EH. Qualitative and mixed methods provide unique contributions to outcomes research. Circulation. 2009;119:1442–52.
Donaldson C. Eliciting patients values by use of ‘willingness to pay’: letting the theory drive the methods. Health Expect. 2001;4:180–8.
Hansen HP, Draborg E, Kristensen FB. Exploring qualitative research synthesis: the role of patients' perspectives in health policy design and decision making. Patient. 2011;4:143–52.
Hauber AB, Fairchild AO, Johnson FR. Quantifying benefit-risk preferences for medical interventions: an overview of a growing empirical literature. Appl Health Econ Health Policy. 2013;11:319–29.
Hauber AB, Gonzalez JM, Groothuis-Oudshoorn CG, Prior T, Marshall DA, Cunningham C, et al. Statistical methods for the analysis of discrete choice experiments: a report of the ISPOR conjoint analysis good research practices task force. Value Health. 2016;19:300–15.
Henshall C, Oortwijn W, Stevens A, Granados A, Banta D. Priority setting for health technology assessment: theoretical considerations and practical approaches. Int J Technol Assess Health Care. 1997;13:144–85.
Hummel JM, Bridges J, IJzerman M. Group decision making with the analytic hierarchy process in benefit-risk assessment: a tutorial. Patient. 2014;7:129–40.
Ijzerman M, van Til J, Bridges J. A comparison of analytical hierarchical process and conjoint analysis methods in assessing treatment alternatives for stroke rehabilitation. Patient. 2012;5:45–56.
Johnson FR, Lancsar E, Marshall D, Kilambi V, Mühlbacher A, Regier DA, et al. Constructing experimental designs for discrete-choice experiments: report of the ISPOR conjoint analysis experimental design good research practices task force. Value Health. 2013;16:3–13.
Loukanova S, Molnar R, Bridges J. Promoting patient empowerment in the health care system: highlighting the need for patient-centered drug policy. Expert Rev Pharmacoecon Outcomes Res. 2007;7:281–9.
McFadden D. Conditional logit analysis of qualitative choice behavior. In: Zarembka P, editor. Frontiers in econometrics. New York, NY: Academic; 1974.
MDIC. A framework for incorporating information on patient preferences regarding benefit and risk into regulatory assessments of New Medical Technology. 2015.
Nelson EC, Eftimovska E, Lind C, Hager A, Wasson JH, Lindblad S. Patient reported outcome measures in practice. BMJ. 2015;350:g7818.
Rotter J, Foerster D, Bridges J. The changing role of economic evaluation in valuing medical technologies. Expert Rev Pharmacoecon Outcomes Res. 2012;12:711–23.
Staniszewska S, Haywood K, Brett J, Tutton L. Patient and public involvement in patient-reported outcome measures: evolution not revolution. Patient. 2012;5:79–87.

Trujols J, Portella MJ, Iraurgi I, Campins MJ, Siñol N, de Los Cobos JP. Patient-reported outcome measures: are they patient-generated, patient-centred or patient-valued? J Ment Health. 2013;22:555–62.

Vogt F, Schwappach D, Bridges J. Accounting for tastes: a German perspective on the inclusion of patient preferences in health care. PharmacoEconomics. 2006;24:419–23.

Wiering B, de Boer D, Delnoij D. Patient involvement in the development of patient-reported outcome measures: a scoping review. Health Expect. 2016;20:11–23. doi:10.1111/hex.12442.

Part III
Country Approaches and Stakeholder Views

Chapter 19
Australia

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19.1 Introduction

At a national level, the Australian Government has two main HTA committees that make recommendations to the health minister/government for public funding of health interventions. These are the Pharmaceutical Benefits Advisory Committee (PBAC) for medications and vaccines and Medical Services Advisory Committee (MSAC) for medical services, diagnostics, and devices. Both have at least one patient representative (sometimes two) on their expert committees and provide opportunities for public and patient input, either as individuals or as organized support groups. Consultation takes place at different stages of the HTA process. PBAC has an input process at appraisal stage, whereas for medical services input is sought at the protocol or scoping stage with specific questions to address. While other HTA activities occur outside these processes—including at a state, hospital, and private health insurance level—in this chapter we focus on review and reform in patient involvement centered primarily on these two programs.

19.1.1 HTA and National Public Reimbursement

Nationally, health technologies are reimbursed through the Pharmaceutical Benefits Scheme (PBS) and the Medicare Benefits Schedule (MBS). In 1954, under the National Health Act (1953), the PBAC was established as an independent expert

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body appointed by the health minister of the Australian government to advise the minister on the listing of new medicines on the PBS for government subsidy. The Act requires that when considering a proposal for listing of a new medicine on the PBS, the comparative clinical effectiveness and cost effectiveness are assessed to ensure the community is receiving "value for money" (Lopert 2009). MSAC was established some years later, in April 1998, as a non-statutory committee to advise the Minister on the public subsidy of medical services. The model was similar to PBAC in that comparative evidence was to be assessed, but in this case relating to safety and financial impact in addition to clinical effectiveness and cost-effectiveness.

19.2 Impetus for Patient Involvement

The largest reform in Australian health policy was arguably the establishment, in 1984, of Medicare, Australia's universal health system. Around this time the Consumers Health Forum (CHF) was also born. The CHF was set up as a national body to provide patient and community perspectives in health policy (Bastian 1998). Indeed, over the years the CHF has been, and continues to be, a key organization and driver of patient involvement in Australian HTA decision-making. Much of the policy discourse around patient involvement in Australian HTA is about improving health outcomes and increasing transparency of decision-making (Department of Health and Ageing 2009).

In recent years the focus on early access to new health technologies has increased. Patients (and patient organizations) also want greater opportunities to communicate their experiences, particularly in situations where there is a paucity of clinical evidence or where cost-effectiveness has not been demonstrated. Such cases include the assessment of trastuzumab for breast cancer (MacKenzie et al. 2008) by the PBAC, which saw well-organized patient groups successfully advocating for reimbursement of treatments. This has motivated other patient groups to follow similar strategies, particularly around treatment for rare diseases. This has meant that the Australian government needs to manage patient expectations within an evidence-based framework while addressing increasing expenditure associated with advances in technology.

In 2004, a research report was commissioned to look at the impact of such developments (Productivity Commission 2005). This was followed by the 2009 Australian HTA Review (Department of Health and Ageing 2009), the 2015 Efficiency in Health Report (Productivity Commission 2015), and more recent reviews around specific MBS (Department of Health 2015) and PBAC processes (Parliament of Australia 2015). Each review makes recommendations in respect to increasing transparency and more strongly structuring patient involvement in HTA used for decision-making.

19.3 Patient Participation

The main approaches to patient involvement in Australian HTA include the appointment of patient representatives on the PBAC and MSAC and call for input by individual patients, their caregivers, patient groups, and others. Both committees have incorporated a position for at least one patient representative over the last 15 years. Until recently, all patient representatives on the committees have been nominated by the CHF. In 2008, the CHF was commissioned by the Department of Health and Ageing to prepare a number of patient impact statements for the PBAC which allowed patients to describe how a condition affected their daily lives and the impact on caregivers. The funding for this however was not sustained.

Currently for the PBAC, the agenda is made public 6 weeks prior to the committee meeting with comments to agenda items invited through the PBAC and PBS websites. The agenda is based on prepared submissions by pharmaceutical industry. Comments received through public consultation are reviewed and presented by the patient representative on the committee for consideration at PBAC meetings. While this process allows individual patients, patient groups, and others to provide comments, the use of the online template is optional, and patients are not provided with details regarding the evidence that is submitted to PBAC nor are they provided with feedback as to the usefulness of their comments to the decision-making process. The public summary documents produced by PBAC to inform stakeholders on the rationale for specific PBAC recommendations acknowledge the patient input received but lack detail about the content of the input or how it influenced the conclusions.

The MSAC process invites public comments at the protocol development stage. This was a change following the Australian HTA Review in 2009, which saw the establishment of a Protocol Advisory Sub-committee, public consultation on protocols via an online questionnaire, and the introduction of patient impact statements for presentation to MSAC. One of the roles of the CHF has been to notify their member base of upcoming reviews so as to encourage submissions of statements and comments. Any feedback received from the public and patients during the evaluation process are then summarized in the public summary document that outlines the considerations and recommendation regarding reimbursement under the MBS.

19.4 Use of Formalized Patient-Based Evidence

Around the time of the 2009 HTA Review, a new framework was also established to evaluate, and potentially disinvest, items already listed on the MBS. The MBS Quality Framework (Department of Health and Ageing 2010)—later called the Comprehensive Management Framework (CMF)—aimed to develop a systematic approach to reviewing existing items that had previously not been formally evaluated. The approach included a review of the evidence base, along with a detailed

assessment of the use of the item and a focus on stakeholder views and perspectives. As part of this, the Department of Health commissioned a number of demonstration reviews to be undertaken by organizations with HTA methodological expertise. Most reviews included a separate section describing and analyzing the literature on patient values and preferences for the particular medical service. This was the first time that such an explicit evidence-based process was followed for capturing patient perspectives in HTA reports. However, subsequent reviews under the CMF were not required to systematically look at this type of evidence and needed to follow a faster process. This work now continues under the MBS Review Taskforce as a rapid and clinically led process (Department of Health 2015).

A second patient involvement initiative was trialed within the MSAC decision-making process. In 2012, the CHF was awarded a short-term grant by the Department of Health to develop a formalized methodology to collect and analyze patient stories This was the "Real People Real Data" project, which generated a tool kit and story wheel for use in health decision-making (Consumer Health Forum of Australia 2013). The tool was piloted for use in a number of organizations, including the MSAC, and the evaluation found benefits for both patients and decision-makers. While the tool kit is available freely for use, it has not been imbedded into either the MSAC or PBAC processes.

19.5 Recent Developments

Developments continue up to the present time. One is the implementation of consumer hearings as part of the PBAC process. These hearings were introduced during the 2015 Senate Committee inquiry on the availability of new innovative and specialist cancer medicines. The inquiry heard that patients wanted a more active role in the PBAC process, particularly for technologies where there was a lack of evidence. The inquiry also made public that for one particular PBAC meeting, the patient representative needed to review and analyze over 2000 individual submissions received as part of the public consultation process (Parliament of Australia 2015). It was at this particular PBAC meeting that a new consumer hearing process was piloted.

The consumer hearings were initiated as a means to give patients another avenue for input. The hearings are not held for all items being considered by the committee but rather for those identified by the PBAC as needing further interpretation around their benefits and harms (Ley 2015). For these selected items, patient groups and/or representative patients are invited to attend a face-to-face meeting with members of the PBAC. Items have included technologies for hepatitis C, melanoma, chronic lymphoma, and inflammatory bowel disease.

The MBS review announced in 2015 also has the potential to offer further opportunities for patient involvement within the assessment of medical services. There is a need to involve patients in a "reshaping" of the Medicare system, which will

require decisions around which technologies should no longer be subsidized on the MBS (Department of Health 2015). The government has earmarked substantial funds to undertake this process, and the CHF is engaged in nominating representatives on clinical working committees.

19.6 Challenges and Future Needs

There are a number of challenges to strengthening patient involvement in the HTA system. One is the way in which HTA decision-making is structured in Australia. Any changes to the process, including how patients are involved, need to have ministerial approval and may require changes in legislation. For example, legislative change would be required for the PBAC to explicitly consider social values in addition to effectiveness and cost-effectiveness. Ministerial approval was needed to run the PBAC consumer hearings. Health policy is politically charged in Australia, and as such changes are time-consuming and often constrained by resources and political sensitivities. This perhaps explains in part why so many recommended reforms with the Australian HTA system have stalled (Productivity Commission 2015).

While other HTA organizations have continued to increase transparency within their systems through improved patient involvement, Australian HTA suffers from a lack of dedicated resources to support patient involvement initiatives. Such support could be used for patient training, developing plain language information on the Australian HTA system and where opportunities exist for patient input, clarity on the purpose of this input, and on what decision-makers want to know from patients and why (Wortley et al. 2016). Including patients earlier in the assessment and having a greater number of patient organizations in the HTA process and at different points have also been suggested (Lopes et al. 2016).

The absence of detailed methodological guidance in this area also is a key barrier to systematically including patient-based evidence in Australian HTA reports. As part of systematizing the inclusion and formal assessment of evidence on patient values, experiences, and preferences, the PBAC and MSAC guidelines would benefit from a requirement that literature on patient values and preferences be included within both pharmaceutical industry submissions and assessment reports. This would provide additional background to the patient and public comments received during the consultation processes.

Finally, there is a real need for formal, publically accessible evaluation of patient involvement initiatives, including the current PBAC consumer hearings. The only formal evaluation undertaken to date has been within the confines of the "Real People Real Data" CHF project, funded by the Department of Health (and therefore a requirement of the funding) (Tong 2014). Patient involvement could be strengthened by a better link between efforts to seek patient input into HTA activities, information on what and how that input was utilized, and meaningful involvement in PBAC and MSAC and MBS processes.

19.7 Conclusions

The Australian HTA system has long acknowledged the role of patients in decision-making. The challenge in the coming years will be in identifying which processes to improve patient involvement are preferred across the various stakeholders and how they are able to be utilized in committee decision-making. The Australian health system in 2016 is experiencing an unprecedented time of review, and this current cycle of review offers the best opportunity yet to reform and strengthen patient involvement in the Australian HTA processes.

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References

Bastian H. Speaking up for ourselves: the evolution of consumer advocacy in health care. Int J Technol Assess Health Care. 1998;14:3–23.

Consumer Health Forum of Australia (CHF). Real people, real data. 2013. https://ourhealth.org.au/content/real-people-real-data-toolkit#.WBaHq9J97IU. Accessed 31 Oct 2016.

Department of Health. Medicare benefits schedule review. 2015. http://www.health.gov.au/internet/main/publishing.nsf/content/mbsreviewtaskforce. Accessed 17 July 2016.

Department of Health and Ageing. Review of health technology assessment in Australia. Commonwealth of Australia, Canberra. 2009. http://www.health.gov.au/internet/main/publishing.nsf/Content/hta-review-report.

Department of Health and Ageing. Development of a quality framework for the medicare benefits schedule. 2010. https://www.chf.org.au/pdfs/chf/DoHA_Information_Paper_Development_of_a_Quality_Framework_for_the_MBS_Discussion_Paper.pdf. Accessed 12 July 2016.

Ley S. More consumer consultation in PBS process. 2015. http://www.health.gov.au/internet/ministers/publishing.nsf/Content/health-mediarel-yr2015-ley016.htm. Accessed 12 Feb 2016.

Lopert R. Evidence-based decision-making within Australia's pharmaceutical benefits scheme. Issue Brief (Commonw Fund). 2009;60:1–13.

Lopes E, Street J, Carter D, Merlin T. Involving patients in health technology funding decisions: stakeholder perspectives on processes used in Australia. Health Expect. 2016;19:331–44. doi:10.1111/hex.12356.

MacKenzie R, Chapman S, Salkeld G, Holding S. Media influence on Herceptin subsidization in Australia: application of the rule of rescue? J R Soc Med. 2008;101:305–12.

Parliament of Australia. Availability of new, innovative and specialist cancer drugs in Australia. 2015. http://www.aph.gov.au/Parliamentary_Business/Committees/Senate/Community_Affairs/Cancer_Drugs/Report. Accessed 2 July 2016.

Productivity Commission Efficiency in Health, Commission Research Paper, Canberra. 2015. http://www.pc.gov.au/research/completed/efficiency-health/efficiency-health.pdf, Accessed 3 Aug 2016.

Productivity Commission. Impacts of advances in medical technology in Australia. Research Report, Melbourne. 2005. http://www.pc.gov.au/inquiries/completed/medical-technology/report/medicaltechnology.pdf. Accessed 3 Aug 2016.

Tong A. CHF real people, real data project–pilot phase evaluation. 2014. https://ourhealth.org.au/sites/default/files/docs/chf_rprd_external_evaluation.pdf. Accessed 17 July 2016.

Wortley S, Wale J, Grainger D, Murphy P. Moving beyond the rhetoric of patient input in health technology assessment deliberations. Aust Health Rev. 2016; doi:10.1071/AH1521.

Chapter 20
Brazil

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20.1 Introduction

This chapter describes the activities that are being developed to improve the mechanisms of patient and public involvement in the Brazilian HTA process. In recent years, there has been ongoing analysis and discussion regarding how to introduce patient and public participation into HTA processes in the Brazilian Public Health System (SUS). Community participation is one of SUS's guidelines stated in the Federal Constitution (Brazil 1988) and one of the principles outlined in Organic Health Laws (Brazil 1990a, 1990b). In 2011, the National Committee for Health Technology Incorporation (CONITEC) was created, and the participation of civil society in the HTA process was formalised. In addition, one of the CONITEC's responsibilities is to promote actions motivating social participation (community or individual involvement in decision-making) in this process.

20.2 Processes for Involvement

CONITEC assesses all types of health technologies. According to Law n. 12,401 (Brazil 2011), public participation in HTA occurs through participation of a member of the National Health Council (CNS), who represents citizens and users of SUS. Public participation is also achieved by public consultation on all recommendations and by public hearings in relevant cases.

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20.2.1 Public Consultations

All recommendations issued by CONITEC are open to public consultation for 20 days. In addition to representation in the plenary session through the CNS, the public consultation enables patients, caregivers, patient associations and other stakeholders to participate. Suggestions made in consultation are submitted by a form on CONITEC's website.

In 2012, the year in which CONITEC activities were started, there were 36 public consultations. The most frequent participants were health technology developers and educational institutions. No individual patients participated, only patient associations. Therefore, it was necessary to develop a strategy that would increase and improve the response of SUS users.

So in late 2014, a specific public consultation form was created to capture the perspectives of patients and caregivers related to the technologies assessed. Thereafter, the contributions were submitted in two forms. One for 'technical and scientific contribution' and the other, for 'opinion or experience', captured patients' and caregivers' experiences regarding the technology under assessment.

Since 2015, CONITEC began producing summarised versions of its technical reports prepared in a simplified language to help understanding of the reports and enable SUS users to contribute more easily.

From its creation in 2011 up to December 2015, CONITEC performed 143 public consultations, receiving over 20,000 contributions, 5000 per year on average and more than half of these were from SUS users. The spreadsheets with the suggestions received are available on the CONITEC website. Their compilation and discussion is part of the final technical report, also available on the website (CONITEC 2016).

20.2.2 Surveys

To investigate patient and public needs and preferences, CONITEC began surveys related to its Clinical Protocols and Therapeutic Guidelines (PCDT) in 2015. The first survey used a structured questionnaire on the CONITEC website to gather patient and caregiver opinions about the critical issues that needed to be considered in the PCDT for rare diseases. The survey was disseminated to experts and the target audience (rare diseases patients). CONITEC received 1140 responses, almost all from patient associations, caregivers, patients, and patient guardians. The responses were considered by health and research experts developing the PCDT.

In February 2016, CONITEC performed another survey to gain feedback on the update of all PCDTs published in 2012 and 2013. On this occasion, patients and users had the opportunity to provide information about their diseases and suggestions, such as healthcare improvement, appeal for new technologies and aspects that in their perspective were critically needed to be addressed in the PCDT. In the new

PCDT format, patients and experts will be able to participate in the development of the PCDTs, and there will be a new section with information for patients.

20.2.3 Communication and Transparency

Simple and effective strategies have been developed to support communication and transparency, which are essential ingredients for credibility and adherence of patients and professionals to CONITEC's recommendations.

In 2014, CONITEC began publicising its consultations through social networks, website and email lists aiming to reach the interested audience and ensure a greater participation. People can register on the website to receive information in a monthly newsletter. This has increased public participation of over 400% in the number of annual suggestions: from 2584 in 2014 to 13,619 in 2015.

The quality of suggestions has also been improving. In 2014, CONITEC identified the need to simplify and construct forms for the target audience. The use of simpler forms better targeted to aspects of patient or caregiver experiences improved the benefit of these contributions in the CONITEC's plenary session discussions, increasing the quality of the Brazilian HTA process.

The institutional website was elected as the platform for communication and transparency initiatives. Among other information, it offers a list of all technologies analysed, agendas of plenary sessions, past and ongoing public consultations, final decisions, specific legislation, National List of Essential Drugs of the SUS, and all published PCDTs.

A plain language guide was developed to explain how to participate in the assessment and incorporation process of health technologies and to improve understanding, increase the skills of non-professionals contributing and providing guidance for patient and public contributions.

20.2.4 Patient Representation in the Plenary Session

Some HTA topics assessed by CONITEC in 2014 and 2015 required participation of patient representatives from associations in the plenary session, aiming to solve issues related to the use of technology (e.g. fingolimod for the treatment of multiple sclerosis and budesonide 200 mcg/formoterol 6 mcg in aerosol suspension for the treatment of asthma). This participation brought information from the unique expertise of patients about the issues discussed. It is necessary to enhance this mechanism by identifying the best participation method.

20.3 Future Plans

Some strategies that may be implemented to support patients to participate in CONITEC assessments in the coming years are:

(a) Participation of patient representatives in the CONITEC plenary session for all HTAs
(b) Training and support for patients through targeted meetings
(c) Enhancement of analysis of patient and public suggestions received through the public consultation
(d) Implementation of the pilot project 'media doctor', aiming to follow up and analyse the news published by the national media regarding HTA and CONITEC, to accurately inform patients and the public about HTA

The participation contexts still pose great challenges to be solved in the next years. For patients to be more involved in HTA in Brazil, policy makers and those supporting them must carefully plan and assess the strategies to be used, since the participation is a political construct to be improved, reoriented, and understood in its various contexts, such as that of HTA.

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References

Brazil. Brazilian Federal Constitution. Brasília: Congress Press; 1988.
Brazil. Law n. 8,080. Brasília: Federal Official Gazette of Brazil; 1990a.
Brazil. Law n. 8,142. Brasília: Federal Official Gazette of Brazil; 1990b.
Brazil. Law n. 12,4011. Brasília: Federal Official Gazette of Brazil; 2011.
CONITEC. Participação social. 2016. http://conitec.gov.br/index.php/participacao-social. Accessed 24 Nov 2016.

Chapter 21
Canada

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21.1 Introduction

HTA in Canada takes place at the hospital, regional, provincial/territorial and pan-Canadian levels. Each HTA programme has a different remit, depending on stakeholder needs, and can include assessment of medicines, medical devices, diagnostics, procedures and health and social service programmes. Canadian HTA organisations share common goals for patient involvement but have developed different approaches to achieve them that fit within available resources, timelines and expertise. In this chapter, due to the large number of programmes, we describe patient involvement within a subset that represents HTA at different jurisdictional levels. The included examples are not an exhaustive representation of patient involvement in HTA in Canada; however, they should provide a comprehensive description of the range of strategies used. We begin by describing patient involvement strategies across seven HTA organisations, as summarised in Tables 21.1 and 21.2. We compare strategies and highlight unique features and challenges within each programme. We close the chapter with a focus on how some Canadian HTA organisations are evaluating and accordingly adapting their patient involvement strategies.

21.2 Patient Involvement Strategies

Patient involvement strategies used within Canadian HTA organisations fall into five broad categories: stakeholder feedback, use of patient input templates, synthesis of published literature, interviews and focus groups and committee participation.

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Table 21.1 Description of patient involvement within CADTH HTA programmes

	HTA type	Number of HTAs per year	PI strategy(s)	How patient perspectives are used	Unique features

CDR[a]	STA (drug)	40–45	• Input submitted via template by patient groups and individual patients if no patient group
			• Two public, no patient, expert committee members
				• To identify patient-important outcomes and contextualise clinical trial results
				• Summarised for deliberation by public member
					• Tailored feedback letters sent post-review outline
					  • What was most useful
					  • Suggestions for future submissions

pCODR[b]	STA (oncology drug)	20–25	• Stakeholder feedback on draft report and recommendations • Input submitted via template by registered patient groups and individual patients if no patient group • Three patient expert committee members	• To identify patient-important outcomes and contextualise clinical trial results • Summarised for deliberation by patient member	• Stakeholder feedback on initial recommendations for deliberation by expert committee and reconsideration of recommendation, if appropriate
Optimal use	STA or MTA (drug class, device, procedure)	6–8	• Stakeholder feedback on included studies, draft report and recommendations • Input submitted via template by patient groups and individual patients if no patient group (drug) • Literature synthesis (device/procedure) • One or two public, no patient, expert committee members	• To identify patient-important outcomes and increase understanding of how drugs are used outside of clinical trials (drug) • Summarised for deliberations by public member (drug) • Used in committee deliberations with support of deliberative framework	• Multiple opportunities for patient groups and individuals to participate (drug) • Literature synthesis providing comprehensive and rigorous evidence (device/procedure)
Scientific advice	Drug clinical trial design advice	6–8	• Individual patient interview	• Contributes to advice given to company on planned trials, including comparators, primary and secondary outcomes and measurement tools	• Interviewed participants are paid honoraria • Extensive interactions with participants

CADTH Canadian Agency for Drugs and Technologies in Health, *CDR* Common Drug Review, *MTA* multiple technology assessment, *N/A* not applicable, *pCODR* Pan-Canadian Oncology Drug Review, *pERC* pCODR Expert Review Committee, *PI* patient involvement, *STA s*ingle technology assessment

[a]See https://www.cadth.ca/about-cadth/what-we-do/products-services/cdr/patient-input

[b]See https://www.cadth.ca/pcodr/patient-input-and-feedback

Table 21.2 Description of patient involvement in other Canadian HTA programmes

	HTA type	Jurisdiction	Number of HTAs per year	PI strategy(s)	How patient perspectives are used	Unique features
BC PharmaCare[a]	STA (drug)	Provincial	50	• Input submitted via online survey by BC patients, caregivers or patient groups	• To contextualise clinical results and understand implementation considerations • Summarised for deliberation by a public member	• Individuals participate • Online survey
Health Quality Ontario	STA (device, procedure)	Provincial	12, with 3 to 5 prioritised for PI	• Stakeholder feedback on draft reports and recommendations • PI strategies are prioritised and can include literature syntheses, interviews, focus groups and/or a survey	• Used in committee deliberations in support of deliberative framework	• Plain language summary to encourage public understanding of health research • Goal to facilitate new technology uptake
Ontario public drug programs (OPDP)[b]	STA(drug); MTA (drug class)	Provincial	60–70[c]	• Input submitted by registered patient groups via a template • Two patient expert committee members	• To understand need for new drug, contextualise clinical results and understand implementation considerations • Summarised for deliberation by patient member	• Consistent with other PI strategies in Canada

(continued)

Table 21.2 (continued)

	HTA type	Jurisdiction	Number of HTAs per year	PI strategy(s)	How patient perspectives are used	Unique features
Centre hospitalier universitaire (CHU) de Quebec	STA (device, procedure)	Hospital	10 HTAs 20 rapid responses or updates	• Tailored strategy that can include interviews or focus groups • Up to three patient HTA working group members • One patient expert committee member	• To contextualise clinical results and understand implementation considerations • Used in committee deliberations	• PI in hospital HTA unit • Formal and theory-driven approach to process development • Focus on evaluation • Targeted and flexible framework
Alberta Health[d]	STA (device, procedure)	Provincial	10	• Tailored strategy that can include literature synthesis, interviews or focus groups • Minimum of two patient expert committee members	• To define scope and context for HTA • Used in committee deliberations to support implementation considerations	• Interviews or focus groups to describe the context in which a technology will be implemented

Institut national d'excellence en santé et en services sociaux (INESSS)	STA, MTA (drug)	Provincial	100 or more drug HTAs 25 other HTAs	• Stakeholder feedback on recommendations (other HTA) • Input submitted by patients and patient groups via a letter (non-template, drug) • Tailored strategy that can include interviews, focus groups or a survey • On request, expert committee meets with patient groups (drug) • Patient advisory committee members (non-standing)	• To understand impact of condition on day-to-day life and QoL (drug) • Used in committee deliberations (drug) • Used throughout project, from scope identification through deliberation (other HTA)	• Individuals participate • Letters of acknowledgement and feedback on how input was used • A variety of methods to obtain patient input (other HTA)

HTA Health technology assessment, *CDR* Common Drug Review, *CADTH* Canadian Agency for Drugs and Technology in Health, *HQO* Health Quality Ontario, *MTA* multiple technology assessment, *PI* patient involvement, *QoL* quality of life, *STA* single technology assessment

[a]See http://www2.gov.bc.ca/gov/content/health/health-drug-coverage/pharmacare-for-bc-residents/what-we-cover/drug-coverage/drug-review-process-results/your-voice

[b]Information accurate as of February 2016. See http://www.health.gov.on.ca/en/pro/programs/drugs/patient_evidence.aspx

[c]Includes both original submissions to OPDP and drugs that have been assessed by CADTH CDR or pCODR

[d]Includes the Institute of Health Economics, the University of Calgary HTA Unit and the Health Technology and Policy Unit at the University of Alberta

21.2.1 Stakeholder Feedback

The earliest example of patient involvement by Canadian HTA organisations was the solicitation of stakeholder feedback. Stakeholders include members of patient groups, individual patients, individual clinicians and members of professional societies, academic groups, pharmaceutical and medical device companies and health authorities. Today, several Canadian HTA programmes solicit stakeholder feedback upon the completion of various milestones, including project scope, list of included studies, draft report and expert committee recommendations. At Health Quality Ontario (HQO), for example, public consultation occurs following the publication of draft reports and recommendations and involves active targeting of patients and patient groups. At CADTH's pan-Canadian Oncology Drug Review (pCODR), programme stakeholders, including patient groups, comment on the pCODR Expert Review Committee (pERC) initial recommendation. pERC then uses this feedback to determine if the initial recommendation is eligible for conversion to a final recommendation without reconsideration. While it is used in limited circumstances, this step allows recommendations to be finalised sooner. A key criterion is that eligible stakeholders, including patient groups, must reach consensus on the recommended clinical population in the initial recommendation and make no substantive comments. The initial recommendation for enzalutamide for first-line treatment of metastatic prostate cancer, for example, was eligible for early conversion: there was unanimous support from all stakeholders, and the patient group stated that the initial recommendation and reasons were clear and that its input was appropriately considered and reflected (pCODR 2015).

Stakeholder feedback has been categorised conceptually as a consultative type of public engagement that is one-way (Rowe and Frewer 2005). For this reason, stakeholder feedback and other consultative methods are noted to have limitations. Without obligation on the part of the HTA programme to adopt the feedback, and without any decision-making power on the part of patients, stakeholder feedback has been characterised as a tokenism-level strategy (van Thiel and Stolk 2013). While concerns about tokenism are justified, stakeholder feedback represents a feasible and potentially useful opportunity for input into an HTA, particularly if coupled with other patient involvement strategies and a commitment by the organisation to carefully consider the feedback received. Canadian HTA organisations use stakeholder feedback extensively and strive to ensure efficient processes and infrastructure are in place to prompt the right stakeholders to provide the right sort of feedback at the right time.

21.2.2 Patient Input Templates

In 2007, the Conseil du Médicament (now INESSS: Institut national d'excellence en santé et en services sociaux) began accepting unstructured patient and other stakeholder input as part of its medicines assessment process, typically as letters or

emails. When other Canadian programmes began to accept input from patients in 2010, they standardised this process by developing and implementing a template, adapted from one originating from the Scottish Medicines Consortium. Canadian HTA programmes now commonly use a template to gather patients' and caregivers' input, although there are variations among programmes in terms of who is able to submit (e.g. patients vs. patient organisations) and how the information is used in assessments and deliberations (see Tables 21.1 and 21.2).

Between 2010 and 2015, 114 patient groups submitted 297 completed patient input templates to CADTH's Common Drug Review (CDR), contributing to 142 reimbursement recommendations (CADTH 2015a). Patient input submitted to CADTH's CDR is also made available to participating federal, provincial and territorial pharmaceutical benefit plans, for their use and local deliberations. Some such drug plans, including the British Columbia (BC) PharmaCare and the Ontario Public Drug Programs (OPDP), additionally collect patient input through their own local process. In Ontario, because patient groups are aware that their submissions to CADTH's CDR are shared with OPDP, some do not submit to OPDP for the same medicine, resulting in on average less than one Ontario-specific submission per medicine. In contrast INESSS, which currently does not use a template approach, can receive up to 50 letters and emails per medicine, although most often receives one or two.

As HTA incorporates wide-ranging and diverse sources of evidence, it is challenging to track the impact of information collected through the patient input template on the assessment or deliberations. Unmet need for the technology under review, as identified through patient input, has occasionally been cited as a reason for a positive reimbursement recommendation by the CADTH Canadian Drug Expert Committee (e.g. asfotase alfa (Strensiq®) (CADTH 2016d), elosulfase alfa (Vimizim®) (CADTH 2016e), ivacaftor (Kalydeco®) (CADTH 2015e)). More frequently, patient input is used by assessors to identify patient-important outcomes to be included in the protocol, prompt consideration of real-world applicability of clinical trial data and offer insights about when the new medicine might be especially useful (Berglas et al. 2016).

Our conversations with programme informants identified some common challenges as either receiving too much or not enough patient input and a lack of adequate resources and time to effectively reach patient groups or promote the strategy. From the perspective of patient groups, challenges include the perception that patient input is not valued at the same level as clinical or economic evidence (Gauvin et al. 2011), feelings of tokenism and the extensive time and expertise required to compile quality submissions with a short time frame and limited budget (Best Medicines Coalition 2014). In response, Canadian HTA programmes continue to adapt their patient input strategies. CADTH's pCODR programme and the Canadian Cancer Action Network (CCAN) have codeveloped an education and support programme to assist patient groups in developing submissions. It includes a detailed guide for completing templates (CADTH-pCODR 2015), narrated slide presentations to describe the pCODR process and provide guidance on collecting and summarising patient input (CADTH 2015b), a cancer medicine pipeline (CCAN 2016),

and a sponsored HTA Patient Engagement Navigator. The Navigator is a free support for the cancer patient community, with responsibility to explore, develop and support a range of opportunities for enhanced patient involvement. This initiative is funded by the Canadian Partnership Against Cancer with in-kind funding from CADTH and CCAN. CADTH CDR employs a full-time patient engagement officer to support patient groups and in 2014 began sending feedback letters to each group submitting input. The letters outline what was most useful within the patient group submission, provide suggestions for future submissions and express thanks and recognition for the group's effort. In a retrospective audit of feedback letters, it was found that patient groups frequently used the specific suggestions in completing subsequent submissions and appreciated the feedback (Rader and Bond 2016).

21.2.3 Synthesis of Published Literature

Some organisations review and synthesise relevant published, often qualitative, literature to incorporate evidence of patients' perspectives and experiences into assessments and deliberations. As further described in Chap. 15, published literature is sought through an electronic database and website search, screened for relevance according to predetermined eligibility criteria, and relevant data are extracted for analysis, much the same as a systematic review of clinical evidence. Depending on timelines, resources and topic-specific needs, different methodological steps are pursued in more or less detail by different programmes and for specific HTAs. The data synthesis method may vary depending on the type of literature reviewed and policymakers' needs. For example, a recent synthesis conducted for HQO of how patients with uncontrolled type 1 diabetes perceive their quality of life (Vanstone et al. 2015a) included a qualitative meta-synthesis (Korhonen et al. 2013), while another synthesis conducted by CADTH on the perspectives of people with colorectal cancer regarding mismatch repair deficiency (dMMR) testing (CADTH 2016a) included a thematic synthesis (Harden et al. 2004). The product of any literature synthesis is either a descriptive or interpretive account that is used alongside clinical, economic and other evidence to support deliberations. The type 1 diabetes meta-synthesis, for example, was considered as evidence for the criterion of 'consistency with expected societal and ethical values' by the Ontario Health Technology Assessment Committee within their decision-making framework that also considers evidence for overall clinical benefit, value for money and feasibility of adoption into the healthcare system (Medical Advisory Secretariat 2010). The dMMR thematic synthesis fulfilled the criterion of 'patient preferences' within CADTH Health Technology Expert Review Panel's (HTERP) deliberative framework (CADTH 2015c), which also includes criteria for clinical benefit and harms, economic impact and implementation, legal, ethics and environmental domains.

The use of literature syntheses helps to ensure a range of patients' and caregivers' perspectives regarding the value, impact, needs, preferences and experiences with health technologies are included in assessments and deliberations (Hansen

2008). The dMMR synthesis, for example, was cited as support for a recommendation for universal testing, as patients and their families were found to value knowledge of their dMMR status so they could manage their future risk and engage in preventive behaviours (CADTH 2016b). This synthesis also highlighted several implementation considerations, including the need for education for patients, families and their healthcare providers and the need for adequate genetic counselling capacity (Weeks et al. 2016).

Literature syntheses represent a relatively efficient process compared to primary data collection and are widely viewed as a more robust form of evidence (Hansen et al. 2011; Facey et al. 2010). For some topics, however, there could be too few relevant published studies available for synthesis or too few relevant to the Canadian context, reflecting a public payer system, unique vulnerable populations, local clinical care pathways and currently available treatments. Furthermore, developing literature syntheses requires specialised skills, for example, in searching, appraising and synthesising qualitative research, and HTA programmes require the capacity to incorporate them into their process. A rigorous synthesis requires iterative review and analysis of the data, which can take a long time and be difficult to fit within a tight HTA timeline.

21.2.4 Interviews and Focus Groups

Another common strategy to obtain evidence related to patients' perspectives is the use of interviews and focus groups. Programmes that use these strategies often recruit patients or their caregivers to collect information regarding what it is like to live with a condition and the realised benefits or harms of using a technology. Of note, due to time constraints and differing goals, Canadian HTA programmes most often do not conduct interviews or focus groups within the context of a qualitative research study, which would encompass a theoretical framework to guide sampling decisions, data collection and analysis and have the goal to produce a new form of knowledge. Instead, interviews and focus groups are most often conducted to collect descriptive information, which is then summarised thematically with the pragmatic intent to inform assessments and deliberations.

As part of the Alberta Health Technologies Decision Process, key stakeholders, including patients and their caregivers, and also clinicians, administrators and policymakers, are recruited through convenience and snowball sampling to participate in semi-structured interviews to explore the feasibility and context of implementing a new technology. In an HTA of hepatitis C screening, 14 telephone interviews with diverse informants across three cities were conducted by trained interviewers (Leggett et al. 2016). Data were analysed to develop key themes that described the current screening, diagnosis and treatment context, including the burden of living with hepatitis C virus, barriers to screening and suggestions for increasing capacity for screening, diagnosis, treatment and support. With a broader focus, HQO pursues interviews or focus groups, often with the assistance of contracted academic health

researchers, when published literature is too limited for a meaningful synthesis. One example is HQO's exploration of women's experiences and values related to publicly funded non-invasive prenatal testing (Vanstone et al. 2015b). Thirty-eight women were interviewed by trained interviewers, and a constructivist grounded theory approach was used to guide data collection and analysis. The analysis demonstrated that perceptions of timing, accuracy and risk influence women's decision-making and that these perceptions conflict with the manner in which publicly funded non-invasive prenatal testing was being offered in this jurisdiction.

CADTH's Scientific Advice Programme is unique in Canada, with the mandate to provide early advice to pharmaceutical companies about clinical trial design from an HTA perspective relevant to the Canadian setting. As part of this programme, CADTH staff interview individual patients and their caregivers to obtain details on symptoms, impact of symptoms, treatment experiences and thoughts on the proposed trial design, with a particular focus on health-related quality of life outcomes. Potential participants are identified through established patient groups and clinical contacts, and those who have broad exposure to other patients' viewpoints are recruited. Insights from the interview(s) are used to confirm or identify patient-important outcomes and relevant comparators and support the advice given by CADTH. A confidential summary of the patient or caregiver interview(s) is shared with the pharmaceutical manufacturer.

Challenges exist with the use of interviews and focus groups within HTA. Recruiting suitable participants who are willing and able to participate can be time-consuming and introduce delays. Expertise with qualitative interviewing, transcription and data analysis are also required. Further, submission to and approval from an ethics review board if required might not line up with HTA timelines (Vanstone and Giacomini 2016).

21.2.5 Committee Participation

Most Canadian HTA programmes include patient or public members on project-specific working groups established for an HTA, on standing expert or advisory committees that make recommendations, or on both. For example, CHU de Quebec establishes a multidisciplinary working group including up to three patient members that is responsible for undertaking the assessment. Recruitment varies depending on the HTA topic. In a 2013 HTA of alternatives to seclusion and restraint for psychiatric patients, patient members were recruited through mental health organisations (Gagnon et al. 2013). In this instance, the direct involvement of patient representatives helped to integrate patients' perspectives during discussions, obtain feedback on HTA results and enhance credibility and confidence in results (Gagnon et al. 2015). At INESSS, while not standing members, patient representatives are invited to provide input to project-specific advisory committees created for most non-medicine HTAs. As with CHU de Quebec, recruitment varies depending on the HTA topic. If a patient group exists and has members, that group may be asked to

help recruit representatives. Otherwise, representatives may be recruited through postings by health professionals in their hospital or clinics or through INESSS's social media, website or newsletter.

In some jurisdictions, patient representatives are members of expert committees that have the mandate to provide recommendations on the funding and use of health technologies, while in other jurisdictions patients' perspectives are presented by public members during expert committee deliberations. At CADTH pCODR and OPDP, two patient members sit on the expert committees with the additional responsibility of summarising and presenting patient group input during deliberations for each medicine under review. Within other programmes (e.g. CADTH Health Technology Expert Review Committee and CADTH Canadian Drug Expert Committee, HQO and BC PharmaCare), public members serve the same role (CADTH 2011a; CADTH 2013; Public Appointments Secretariat 2015; BC PharmaCare 2010). In all cases, both patient and public members are provided the same rights and are held to the same terms of reference and conflict of interest guidelines as other expert committee members (CADTH 2011b; BC PharmaCare 2009; Public Appointments Secretariat 2016).

While there are variations across programmes, the inclusion of patient or public members helps ensure that patient relevant information is included within deliberations. An important challenge, however, is identifying people to fill this role, and most committees have developed criteria by which to recruit and select members. There is general agreement that patient or public committee members should represent the broad perspective of the people who may use or have a need for a health technology under review. To be meaningful, committees must deliberate with knowledge of a variety of patients' experiences, including experiences over time and within different aspects of their lives. At CADTH pCODR, for example, patient members are selected for the expert committee based on their demonstration of personal knowledge of, experience with and understanding of issues related to cancer and its management, among other qualifications (CADTH 2015d). Patient or public members can effectively bring this perspective to deliberations; however, in order to do so they must also have the confidence to express their opinions and encourage a discussion that reflects the patient perspective as part of a highly technical conversation with other members, clinical experts, researchers and decision-makers. Other committee members, especially the committee chair, play an important role in establishing an egalitarian environment such that patient and public members can achieve this purpose (Thomas and Meredith 2012).

21.3 Evaluation of Patient Involvement in Canada

As a relatively new strategy within HTA, best practices in patient involvement have yet to be established (Gauvin et al. 2014). Ongoing evaluation, reflection and refinement of patient involvement programmes are important to ensure patients are involved efficiently and their participation adds value. In Canada, most programmes

record key metrics to measure the extent of participation, such as the volume of patient input received and stakeholders providing feedback. As indicators of participation within established programmes, these metrics do not reflect the value or impact of patient involvement and how patient relevant information might be used, or not, within assessments or deliberations.

Some programmes have undertaken more formal evaluations. In 2012 CADTH contracted an independent consulting firm to evaluate the patient input process within the CDR. The evaluation included a document review, key informant interviews, a survey and a literature search to evaluate the process in three dimensions: philosophy and goals, design and execution. Results demonstrated that the programme was well developed, and, with few exceptions, stakeholders felt it to be important and that it provided unique and relevant information to the medicine review process. Nonetheless, several recommendations for improvement emerged and were subsequently implemented, including the need to clearly define programme objectives, develop a framework and be transparent in how patient input is used, increase programme awareness, improve communication with patient groups, and offer training and support for patient groups to develop useful submissions (SECOR 2012). These recommendations prompted several changes to the patient input process, such as developing information sessions and awareness strategies, hiring a dedicated staff member, holding training sessions and developing a process for individual patients to provide input when a patient group does not exist. To further enhance patient involvement, the Patient Community Liaison Forum (CADTH 2016c) was formed to assist in identifying priorities for patient input-related activities, share learnings and experiences about involving patient groups and facilitate information sharing with and from patient groups. While these recommendations were developed specifically for the CDR programme 4 years ago, their continued relevance to CADTH and other programmes remains apparent.

In another formal evaluation, CHU de Quebec researchers evaluated patient involvement within a specific HTA (Gagnon et al. 2013). In this HTA, patients participated in a working group that developed and managed the project and additionally participated in focus groups to provide their perspectives and experiences to inform the assessment. Data were collected for the evaluation through semi-structured interviews with a range of people involved in the project. Interviews focused on how people perceived the point of view of patients to have been considered in the HTA and whether and how patient involvement changed the assessment or recommendation. Results demonstrated that patient involvement can, among other benefits, help identify critical implementation issues and strategies, clarify input from other stakeholders, inform the development of recommendations that reflect patient needs and ensure recommendations are accessible to the patients and families who will be impacted by them (Dipankui et al. 2015). The evaluation supported the feasibility of these two patient involvement strategies (committee participation and focus groups) and the feasibility of evaluating patient involvement within specific HTAs. Important lessons learned included that, in order to facilitate objectivity, the person conducting the evaluation should ideally be independent and not involved in the assessment, that such a formal and resource intense evaluation is

not possible for all HTAs, and that a more targeted, practical and standardised evaluation strategy is needed. CHU de Quebec researchers are developing such a strategy (Marie-Pierre Gagnon, Population Health and Practice-changing Research Group, CHU de Québec Research Centre, Laval, QC: personal communication, 2016 Jun).

21.4 Lessons Learned

The Canadian experience has made it clear, through both formal evaluations and day-to-day experiences, that the involvement of patients and the inclusion of patients' perspectives in HTA help ensure more informed, patient-centred and relevant decision-making. At the same time, involving patients is difficult. Our conversations with people working within Canadian HTA programmes suggest that effective patient involvement can be resource intensive, requires time to develop productive collaborative relationships built on trust and also requires ongoing effective communication and support. Within fixed and short HTA timelines, and limited budgets, this is not always possible. While there is widespread support for patient involvement across a range of stakeholder groups including patients, HTA organisations, clinicians, HTA researchers and decision-makers, there is also tension between what ideally patient involvement could look like and how practically it can be implemented in this environment. Compromises are needed from both HTA programmes and patients and their families in recognition of this tension.

Further, the Canadian experience suggests a need for flexibility in developing patient involvement strategies that work for a specific HTA programme and each HTA topic. The resources, time and effort dedicated to ensuring effective patient involvement for one topic might exceed what is needed for another topic. There will be situations where intense patient involvement appears warranted but is not possible due to timelines or resource limitations. A one-size-fits-all approach is likely not appropriate. At the same time, it is not appropriate for HTA programmes to suggest that limited timelines and resources must preclude attention towards developing patient involvement. For HTA to be meaningful and reflective of a range of relevant perspectives, patients must be involved and appropriate resources and policies must be in place to support their involvement. Patient involvement strategies must be adapted to ensure the full range of relevant patient perspectives are incorporated in HTA.

21.5 Future Plans

In Canada, building on the lessons learned, HTA programmes are starting to develop flexible frameworks to tailor patient involvement strategies based on programme and topic-specific needs. The goal is for efficient and effective involvement from the perspective of all concerned stakeholders. For example, over 5 years CHU de

Quebec researchers conducted the groundwork to develop such a flexible framework, including a systematic review, local needs assessment, pilot project and evaluation (Gagnon et al. 2014; Dipankui et al. 2015). INESSS is likewise embarking on the development of a methodological framework to support the participation of patients and citizens across all HTA activities. In 2016, a committee was formed to develop and operationalise such a framework. HQO is another example of a programme that implements a flexible strategy to help prioritise—in an environment of limited resources—which HTAs should include a patient involvement component and, of those that do, the most appropriate approach (OHTAC 2015). While HQO commonly includes literature syntheses or analyses of interviews or focus groups as part of its assessments, work is underway to expand patient involvement strategies to include public and patient members (or 'Lived Experience Members') within OHTAC and patient members as expert consultants with the clinical review team. These additional strategies under development demonstrate a willingness to ensure an appropriate involvement strategy is developed in line with the needs for each HTA (Mark Weir, Health Quality Ontario, Toronto, ON: personal communication, 2016 October).

With an ongoing focus on evaluation, flexibility and efficiency, Canadian organisations are well positioned to ensure that patient involvement in HTA in Canada remains meaningful and productive.

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References

Berglas S, Jutai L, MacKean G, Weeks L. Patients' perspectives can be integrated in health technology assessments: an exploratory analysis of CADTH common drug review. Res Involvement Engagem. 2016;2:21. doi:10.1186/s40900-016-0036-9.

Best Medicines Coalition. Health technology assessment in Canada: improving the system and ensuring the patient voice is heard. Position paper. 2014. http://www.bestmedicines.ca/node/168. Accessed 11 Jul 2016.

British Columbia PharmaCare. Conflict of interest guidelines: for the drug benefit review process. British Columbia Ministry of Health Services. 2009. http://www2.gov.bc.ca/assets/gov/health/health-drug-coverage/pharmacare/dbc-coi-guidelines.pdf. Accessed 20 Jun 2016.

British Columbia PharmaCare. Drug Benefit Council–terms of reference. British Columbia Ministry of Health Services. 2010. http://www2.gov.bc.ca/assets/gov/health/health-drug-coverage/pharmacare/dbc-termsofref.pdf. Accessed 20 Jun 2016.

CADTH. Canadian Drug Expert Committee: terms of reference. 2011a. https://www.cadth.ca/media/corporate/corp_committees/CDEC_TOR_e.pdf. Accessed 20 Jun 2016.

CADTH. Conflict of interest guidelines for CADTH expert committee and panel members. 2011b. https://www.cadth.ca/media/corporate/corp_committees/cadth_coi_guidelines_cedc_members_e.pdf. Accessed 20 Jun 2016.

CADTH. CADTH Heath Technology Expert Review Panel: terms of reference. 2013. https://www.cadth.ca/sites/default/files/corporate/corp_committees/hterp/CADTH_Expert_Review_Panel_TOR_revised_July_2013.pdf. Accessed 20 Jun 2016.

CADTH. Common drug review. Ivacaftor (Kalydeco–Vertex Pharmaceuticals Inc.) Indication: cystic fibrosis with R117H mutation. 2015a (CADTH Canadian Drug Expert Review Committee: final recommendation). https://www.cadth.ca/sites/default/files/cdr/complete/SR0430_complete_Kalydeco_R117H_Nov-23-15_e.pdf. Accessed 11 Jul 2016.

CADTH. Updates for patient groups–June 2015. Five years of patient engagement. 2015b. https://www.cadth.ca/news/update-patient-groups-june-2015. Accessed 17 Jun 2016.

CADTH. New resources to help strengthen pCODR patient advocacy group submissions. 2015c. https://www.cadth.ca/new-resources-help-strengthen-pcodr-patient-advocacy-group-submissions. Accessed 11 Jul 2016.

CADTH. Health Technology Expert Review Panel deliberative framework. 2015d. https://www.cadth.ca/sites/default/files/pdf/hterp/HTERP_DFW_e.pdf. Accessed 17 Jun 2016.

CADTH. Help the patient voice be heard–pERC requires a patient member. 2015e. https://www.cadth.ca/news/help-patient-voice-be-heard-perc-requires-patient-member. Accessed 20 Jun 2016.

CADTH. DNA mismatch repair deficiency tumour testing for patients with colorectal cancer: a health technology assessment. 2016a. (CADTH optimal use report). https://www.cadth.ca/sites/default/files/pdf/OP0522_dMMR_Science_Report.pdf. Accessed 21 Oct 2016.

CADTH. Common drug review. Asfotase alfa (Strensiq—Alexion Pharma Canada Corp.). Indication: pediatric-onset hypophosphatasia. 2016b (CADTH Canadian Drug Expert Review Committee: final recommendation). https://www.cadth.ca/sites/default/files/cdr/complete/SR0443_complete_Strensiq-Apr-4-16_e.pdf. Accessed 17 Jun 2016.

CADTH. DNA mismatch repair deficiency tumour testing for patients with colorectal cancer: recommendations. 2016c. (CADTH optimal use report). https://www.cadth.ca/sites/default/files/pdf/OP0522_dMMR_Report_Recommendations.pdf. Accessed 21 Oct 2016.

CADTH. CADTH Patient Community Liaison Forum. 2016d. https://www.cadth.ca/cadth-patient-community-liaison-forum. Accessed 20 Jun 2016.

CADTH. Common drug review. Elosulfase alfa resubmission (Vimizim–Biomarin Pharmaceutical (Canada) Inc.). Indication: mucopolysaccharidosis IVA (morquio A syndrome). 2016e (CADTH Canadian Drug Expert Review Committee: final recommendation). https://www.cadth.ca/sites/default/files/cdr/complete/SR0456_complete_Vimizim_May-26_16.pdf. Accessed 11 Jul 2016.

CADTH-pCODR. pan-Canadian oncology drug review: patient engagement guide. 2015. https://www.cadth.ca/sites/default/files/pcodr/pCODR%27s%20Drug%20Review%20Process/pcodr-patient-engagement-guide.pdf. Accessed 17 Jun 2016.

CCAN, Canadian Cancer Action Network, CADTH pan-Canadian Oncology Drug Review (pCODR). Cancer drug pipeline information for patient advocacy groups. 2016. https://www.cadth.ca/sites/default/files/pcodr/Communications/pCODR-CCAN_HTA_Pipeline.pdf. Accessed 11 Jul 2016.

pCODR, pan-Canadian oncology drug review. pCODR Patient Advocacy Group feedback on a pERC initial recommendation–enzalutamide (Xtandi) for first line mCRPC. 2015.

https://www.cadth.ca/sites/default/files/pcodr/pcodr_enzalutamide_xtandi_1stln_mcrpc_ptfdk_pcc.pdf. Accessed 11 Jul 2016.

Dipankui MT, Gagnon MP, Desmartis M, Legare F, Piron F, Gagnon J, et al. Evaluation of patient involvement in a health technology assessment. Int J Technol Assess Health Care. 2015;31:166–70. doi:10.1017/S0266462315000240.

Facey K, Boivin A, Gracia J, Hansen HP, Lo Scalzo A, Mossman J, et al. Patients' perspectives in health technology assessment: a route to robust evidence and fair deliberation. Int J Technol Assess Health Care. 2010;26:334–40. doi:10.1017/S0266462310000395.

Gagnon MP, Desmartis M, Dipankui MT, Gagnon J, St-Pierre M. Alternatives to seclusion and restraint in psychiatry and in long-term care facilities for the elderly: perspectives of service users and family members. Patient. 2013;6:269–80. doi:10.1007/s40271-013-0023-2.

Gagnon MP, Desmartis M, Gagnon J, St-Pierre M, Gauvin FP, Rhainds M, et al. Introducing the patient's perspective in hospital health technology assessment (HTA): the views of HTA producers, hospital managers and patients. Health Expect. 2014;17:888–900. doi:10.1111/hex.12010.

Gagnon MP, Desmartis M, Gagnon J, St-Pierre M, Rhainds M, Coulombe M, et al. Framework for user involvement in health technology assessment at the local level: views of health managers, user representatives, and clinicians. Int J Technol Assess Health Care. 2015;31:68–77. doi:10.1017/S0266462315000070.

Gauvin FP, Abelson J, Giacomini M, Eyles J, Lavis JN. Moving cautiously: public involvement and the health technology assessment community. Int J Technol Assess Health Care. 2011;27:43–9. doi:10.1017/S0266462310001200.

Gauvin FP, Abelson J, Lavis JN. Evidence brief: strengthening public and patient engagement in health technology assessment in Ontario. McMaster Health Forum. 2014. https://www.mcmasterhealthforum.org/docs/default-source/Product-Documents/evidence-briefs/public-engagement-in-health-technology-assessement-in-ontario-eb.pdf?sfvrsn=2. Accessed 11 Jul 2016.

Hansen HP. Patient aspects in HTA. In: Kristensen FB, Sigmund H, editors. Health technology assessment handbook. 2nd ed. Copenhagen: National Board of Health, Danish Centre for Health Technology Assessment; 2008. p. 104–11. http://sundhedsstyrelsen.dk/~/media/ECAAC5AA1D6943BEAC96907E03023E22.ashx. Accessed 17 Jun 2016.

Hansen HP, Lee A, van Randwijk CB. Patient aspects: a review of fifty-eight Danish HTA reports. Int J Technol Assess Health Care. 2011;27:330–6. doi:10.1017/S0266462311000535.

Harden A, Garcia J, Oliver S, Rees R, Shepherd J, Brunton G, et al. Applying systematic review methods to studies of people's views: an example from public health research. J Epidemiol Community Health. 2004;58:794–800. doi:10.1136/jech.2003.014829.

Korhonen A, Hakulinen-Viitanen T, Jylha V, Holopainen A. Meta-synthesis and evidence-based health care—a method for systematic review. Scand J Caring Sci. 2013;27:1027–34. doi:10.1111/scs.12003.

Leggett L, Coward S, Soril L, Weaver C, MacKean G, Noseworthy T, et al. Hepatitis C screening in Alberta: a health technology assessment. Calgary: Health Technology Assessment Unit, University of Calgary; 2016. http://www.health.alberta.ca/documents/AHTDP-HepatitisC-Screening-HTA-Report-2016.pdf. Accessed 17 Jun 2016

Medical Advisory Secretariat. Decision determinants guidance document: the Ontario Health Technology Advisory Committee (OHTAC) decision-making process for the development of evidence-based recommendations. Ontario Ministry of Health and Long-Term Care. 2010. http://www.hqontario.ca/en/mas/tech/pdfs/2011/guide_decision.pdf. Accessed 17 Jun 2016.

OHTAC, Public Engagement Subcommittee. Public engagement for health technology assessment at Health Quality Ontario—final report from the Ontario Health Technology Advisory Committee Public Engagement Subcommittee. Queen's Printer for Ontario. 2015. http://www.hqontario.ca/Portals/0/documents/evidence/special-reports/report-subcommittee-20150407-en.pdf. Accessed 4 Aug 2016.

Public Appointments Secretariat. Committee to evaluate drugs: agency details. Toronto, ON: Government of Ontario; 2015. https://www.pas.gov.on.ca/scripts/en/BoardDetails.asp?boardID=817. Accessed 20 Jun 2016

Public Appointments Secretariat. Personal and conflict of interest disclosure statement. In: General information. Toronto, ON: Government of Ontario; 2016. https://www.pas.gov.on.ca/scripts/en/generalInfo.asp?#5. Accessed 20 Jun 2016.

Rader T, Bond K. Patient input into the CADTH Common Drug Review: is specific feedback useful to patient groups in preparing patient input submissions? Poster presented at: HTAi 2016 annual meeting: informing health care decisions with values and evidence. 2016 May 10–14; Tokyo.

Rowe G, Frewer LJ. A typology of public engagement mechanisms. Sci Technol Human Values. 2005;30:251–90. doi:10.1177/0162243904271724.

SECOR. CADTH patient input process review: findings and recommendations. CADTH. 2012. https://www.cadth.ca/sites/default/files/pdf/2012_SECOR_Patient-Input-Review_e.pdf. Accessed 4 Aug 2016.

van Thiel G, Stolk P. Background paper 8.5: patient and citizen involvement. Update on 2004 background paper. World Health Organization. 2013 (Priority medicines for Europe and the world. 2013 Update). http://www.who.int/medicines/areas/priority_medicines/BP8_5Stakeholder.pdf. Accessed 11 Jul 2016.

Thomas V, Meredith B. The role of the Chair in patient and public involvement: training and support (2012). In: G-I-N Public Working Group, editor. G-I-N public toolkit: patient and public involvement in guidelines. Guidelines International Network. .2015. pp. 64–75. http://www.g-i-n.net/document-store/working-groups-documents/g-i-n-public/toolkit/toolkit-2015. Accessed 11 Jul 2016.

Vanstone M, Giacomini M. Using qualitative research methods to solicit patient experiences and values for health technology policy-making. McMaster University. 2016 (Presented at the 2016 CADTH Symposium, concurrent session B5). https://www.cadth.ca/sites/default/files/symp-2016/presentations/april11-2016/Concurrent-Session-B5-Meredith-Vanstone.pdf. Accessed 11 Jul 2016.

Vanstone M, Rewegan A, Brundisini F, Dejean D, Giacomini M. Patient perspectives on quality of life with uncontrolled type 1 diabetes mellitus: a systematic review and qualitative meta-synthesis. Ont Health Technol Assess Ser. 2015a;15:1–29. http://www.ncbi.nlm.nih.gov/pmc/articles/PMC4664939. Accessed 17 Jun 2016

Vanstone M, Yacoub K, Giacomini M, Hulan D, McDonald S. Women's experiences of publicly funded non-invasive prenatal testing in Ontario, Canada: considerations for health technology policy-making. Qual Health Res. 2015b;25:1069–84. doi:10.1177/1049732315589745.

Weeks L, Moulton K, Rader T, Garland S, Bond K. Integrating qualitative research into health technology assessment in Canada: the CADTH experience. CADTH. 2016 (Presented at the 2016 CADTH Symposium, concurrent session B5). https://www.cadth.ca/sites/default/files/symp-2016/presentations/april11-2016/Concurrent-Session-B5-Laura-Weeks.pdf. Accessed 17 Jun 2016.

Chapter 22
Denmark

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22.1 Introduction

This chapter presents the Danish HTA model, the premises for patient involvement in Denmark and how that has been operationalised in HTA in Denmark over the past two decades. In Denmark patient involvement in HTA has been developed since the 1980s at both national and regional level. The strategy for patient involvement has explicitly introduced scientific analysis of patient-related aspects to produce patient-based evidence as an essential part of HTA. Secondary research into patients' perspectives has played a major role in patient involvement in HTA in Denmark, and primary research has been carried out when existing research was insufficient to create an understanding of central patient aspects. In addition, patients have participated as stakeholders in HTA processes through representation in stakeholder groups. The rationales for patient involvement in HTA in Denmark are outlined in this chapter. The processes of involvement of patients both as stakeholders and through scientific analysis of patient aspects are discussed. Finally, the impact of the Danish strategy for patient involvement is illustrated, and the future plans for development of patient involvement in a Danish setting are presented.

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22.2 Patient Involvement and the Danish HTA Model

HTA in Denmark was introduced into the political debate at the start of the 1980s. The first HTA report was published in 1980 (Sigmund and Kristensen 2009). In 1982, the National Board of Health was assigned formal responsibility for HTA, and several overviews of HTA design in the Danish context have been formulated since then. The first publication was a short introduction to HTA design that was published in 1984 and revised twice in 1994 and 2000 (National Board of Health 1984; National Board of Health 1994; Danish Institute for Health Technology Assessment 2000). In addition, two comprehensive HTA handbooks were published in 2001 and 2007 (Danish Institute for Health Technology Assessment 2001; Kristensen and Sigmund 2007).

Patient involvement, as defined in Section 22.1.4, can be seen as an integral part of HTA production in Denmark. It has the two distinct strands: (1) patient representatives *participating* as expert stakeholders in the HTA production process and (2) primary and secondary patient-related aspects *research* to produce patient-based evidence. In particular, the second strand has received much attention in the Danish context. Early in the process, the multidisciplinary characteristics of HTA were translated into a model that called for research within all relevant research areas. The model was developed over time and disseminated in the above-mentioned handbooks. Research of patient-specific aspects became a core element in Danish HTA production (Sigmund and Kristensen 2002; Sigmund and Kristensen 2009). The Danish HTA model includes four elements: technology, patient, economy and organisation. In addition to these elements, ethical aspects may also be incorporated in the analyses (Kristensen and Sigmund 2007) (Fig. 22.1).

The model had strong support among regional and local HTA producers and was accepted as the model for HTA in Denmark. For instance, the emphasis on secondary research of patient aspects is included in guidance on performing mini-HTAs locally in hospitals and municipal settings (Danish Centre for Evaluation and Health Technology Assessment 2005; Danish Centre for Evaluation and Health Technology Assessment 2008).

In 2011 the Danish regions took over the formal responsibility for HTA production (with the regional research institution, DEFACTUM, as coordinator of the activities), and funding for activities was given to specific projects. DEFACTUM is still strongly committed to analysis of patient aspects as developed in the Danish HTA model, but the number of produced HTAs has declined. The consequence of this is that local patient participation in HTA has been reduced over the years.

22.3 Rationale for Patient Involvement

The rationale for patient involvement in HTAs is that it contributes to improving healthcare services and ensuring optimal treatment and healthcare conditions for patients. Patients may have important insights into the development of healthcare services to accommodate their needs. Therefore, they may in some HTAs act as partners. In Denmark, two kinds of patient involvement strategies can be identified.

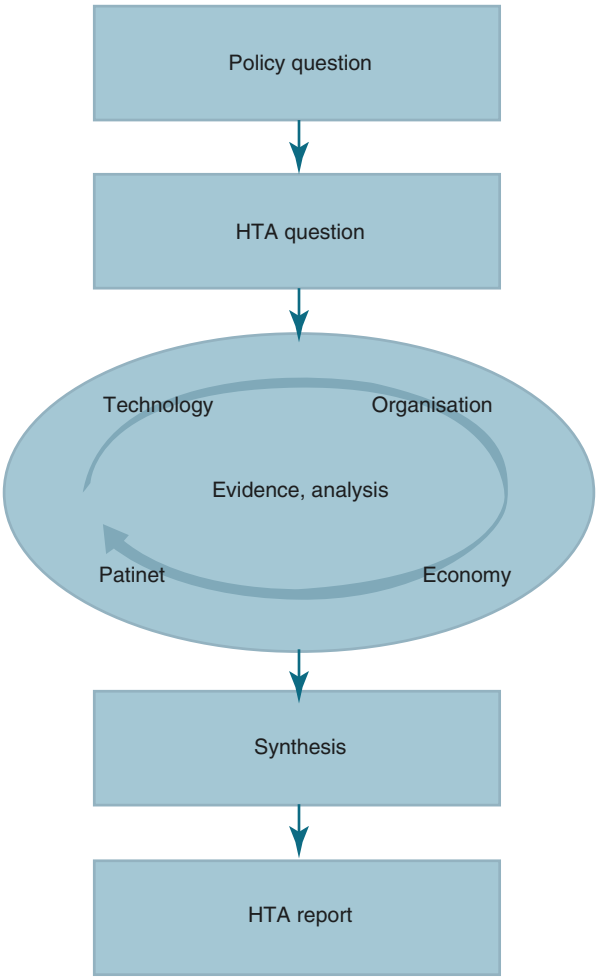

Fig. 22.1 The Danish HTA Model (Kristensen and Sigmund 2007)

One strategy includes patient representatives as expert stakeholders in the HTAs. The participation of stakeholders in general ensures the representation of stakeholder interests, which enhances the legitimacy of the HTA reports and improves the possibility that HTA will be used in policy-making (Palmhøj Nielsen et al. 2009). The Danish HTA handbook calls for stakeholder identification and inclusion and suggests that the following questions should guide which stakeholders to include in HTA:

- Who is the initiator of the HTA?
- Who are the users of the results?
- Who has to accept the results?
- Who pays for the work and the results?

- Who is affected? Who benefits/profits/has drawbacks/risks or is inconvenienced by the results?
- Who has the knowledge and resources or contributes? (Kristensen and Sigmund 2007).

Patient experts represent a central stakeholder group who may be heavily affected by the decisions made out of the results of an HTA report. Therefore, they will need to accept the results if the report is to form a useful basis for policy-making. Expert patients are typically invited into the HTA process at predefined stages to secure a transparent and fair HTA production. The inclusion of expert patients concurrently serves the purpose of 'testing' (1) whether the scope of an HTA is acceptable from a patient perspective and (2) that the HTA includes the relevant aspects from the patients' perspective.

However, during the early stages of development of the Danish HTA Model, it was recognised that stakeholder participation alone was insufficient to assess all relevant patient aspects in HTA. Stakeholder participation should only be considered as a supplement to a more robust perspective, i.e. research of patient-related aspects through a review of existing literature and primary research (Danish Institute for Health Technology Assessment 2001; Kristensen and Sigmund 2007).

Based on this the second strategy of patient involvement requires patient-related aspects to be analysed scientifically as thoroughly, systematically and transparently as other relevant aspects (economy, technology, organisation) of the technology. The latest HTA handbook (Kristensen and Sigmund 2007) suggests that research can contribute knowledge concerning the following aspects:

- Patients' knowledge and experiences of a given technology
- Patients' preferences, needs and expectations of the technology
- Patients' visions and requirements concerning technology, economic aspects and organisation
- How customs, attitudes and traditions influence patients' experiences and preferences
- The importance of the studied technology in question in patients' daily life
- How patients' self-care and/or empowerment resources are best exploited and what opportunities and limitations apply to self-care/empowerment

These aspects should be studied based on existing research related to the relevant technology, with primary research conducted if necessary. The strength of this strategy is that it is based on knowledge from a broad perspective including insights from strategically selected patients and not exclusively from patient representatives who are appointed or volunteered to contribute their knowledge.

Patient aspects are typically investigated and presented in a separate chapter in Danish HTA reports, which allows for a more direct and explicit focus on selected patient aspects. In addition, the results of the research should be included in a concluding part of the HTA report to influence the development of recommendations together with analyses of clinical effectiveness, and organisational and economic aspects (Kristensen and Sigmund 2007; Tjørnhøj-Thomsen and Hansen 2011; Hansen et al. 2011).

22.4 Process for Patient Involvement

22.4.1 Patients as Stakeholders

In Denmark, including patients as stakeholders in HTA refers to the participation of patient representatives (often from patient organisations) in expert groups. These expert groups typically include a variety of relevant experts, e.g. administrative staff, doctors, nurses, health technology developers and patient representatives. The function of an expert group is to give input into HTA, especially in connection with the problem formulation and synthesis phases. In the problem formulation phase, experts are asked to give input to a protocol to ensure that the scope of an HTA is appropriate, that the selected outcomes are exhaustive and that no important elements are omitted.

The HTA handbook recommends that efforts must be made to ensure that the expert group completely understands the project team's proposals and their consequences. In addition, the aim of an expert group is not to achieve a joint decision, but rather to give their input to the HTA producers so that the different viewpoints of the stakeholders can be registered and the producers may consider how the stakeholder input can be integrated (Kristensen and Sigmund 2007).

This strategy for stakeholder involvement places the patient representatives on equal footing with health professionals, administrative staff and health technology developers. In Denmark, there has been little attention given to capacity building and no tradition of educating patients prior to their participation in expert groups. However, HTA producers frequently pay special attention to ensure that patient representatives voice their concerns and input in the larger expert group. Usually representatives selected by Danish patient organisations are skilled in representing patient interests and frequently have substantial experience of interacting with clinicians and government. The general experience is that patient representatives offer valuable input to HTA production and are typically able to contribute to the more focused scope and formulation of an HTA report.

22.4.2 Research into Patient Aspects

To obtain robust evidence about patient aspects, first a systematic literature search on patient-relevant literature is completed, and existing studies are synthesised using rigorous methods (Chap. 15). If more knowledge is needed to answer the research questions, primary research will be undertaken, e.g. interviews, focus group interviews or field studies (Chap. 12, Kristensen and Sigmund 2007).

Patient aspects have been documented to affect the conclusions and recommendations of HTA reports. For instance, this may lead to input on how to organise clinical practices or specific treatments based on patient aspects, how to improve patient information or how to ensure flexibility in patient care due to the understanding of patients as active agents (Hansen et al. 2011).

The following example illustrates how an HTA of screening for abdominal aortic aneurysm (AAA) included an ethical assessment and a systematic review, which resulted in the discussion of patient-related consequences associated with the introduction of a screening programme (Bennetsen et al. 2008).

Patient-Based Evidence for AAA Screening
When screening for AAA, a relatively large group of participants will be diagnosed with a minor AAA. For this group, no treatment is offered but they can have regular follow-up scans. Depending on the size and growth rate of the aneurysm, the patients participate in the monitoring process until the aneurysm requires surgery or until the patient dies. In the HTA report, the patient aspects sought to assess whether the participants' quality of life was affected by participation in screening for AAA. The method applied was a scientific analysis of patient-related aspects based on a systematic literature review. The changes in quality of life were limited. However, the largest changes in quality of life were registered by the group of participants diagnosed with minor AAA. Therefore, the value of a screening programme for this group is questionable. Additionally, the HTA report provided an overview of the patients' experiences during the screening programme. The report concluded that a screening programme potentially benefits patients but may have negative consequences for patients who are not offered immediate treatment and have to participate in regular follow-up scans (Bennetsen et al. 2008).

The HTA resulted in the establishment of a temporary policy-making committee concerning screening in the central Denmark region. The committee's task was to produce recommendations to support regional consideration regarding screening and to contribute to the national debate on the issue (Central Denmark Region 2009).

Another example illustrates the impact on policy of research into patient aspects in a Danish HTA.

Patient-Based Evidence for Gynaecological Cancer Follow-Up
An HTA report on the follow-up of gynaecological cancer patients was requested from clinical and administrative personnel who wanted an investigation of whether the benefits of follow-up ambulatory control justified the resources consumed. The patient aspects were covered through a systematic literature review. The HTA report showed that there was no evidence to show that follow-up programmes improve survival times. Furthermore, the analysis of patient aspects questioned whether the follow-up programmes met the patients' healthcare needs and expectations (Danish Centre for Health Technology Assessment 2009).

This HTA report provided a reason to revise the follow-up programmes for gynaecological cancer patients. Several research projects, including PhD projects, have now been initiated and conducted to generate evidence for changes to the follow-up programmes. The cancer patients' experiences, expectations and needs are central to the research projects. National work on revising the follow-up programmes for gynaecological cancer patients is ongoing. The revision of these programmes aims to include a greater focus on psychosocial elements.

22.5 Impact of Patient Involvement

As indicated, a very important part of Danish HTA reports is research on patient aspects. A review of 58 Danish HTA reports (Hansen et al. 2011) published between 1999 and 2010 showed that:

- Fifty-four reports specifically stated research questions concerning patient aspects.
- Fifty-one reports had a separate chapter concerning patient aspects, which allowed a more direct and explicit focus on the patients' needs, experiences and preferences.
- Two reports had a specific theoretical approach; therefore, patient aspects were integrated into other aspects of the reports. This enabled an investigation into how technology, organisation and patients affect each other.
- All reports included research literature. Twelve reports included systematic literature reviews, while 16 reports browsed the literature.
- Thirty-four reports included primary research using both quantitative and qualitative methods.
- All reports included patient aspects in the conclusions/recommendations of the HTA report.

Compared with a review of the inclusion of patient aspects in INAHTA members' HTA reports (Lee et al. 2009), the above review shows that Danish HTA reports include patient aspects to a greater extent than most and do so in a considered manner (Hansen et al. 2011). This is probably because in the Danish HTA context, analyses of patient aspects are recommended. It is also recommended that researchers base their methodology decisions on clear and explicit reasoning, which improves the quality of research performed on patient aspects (Tjørnhøj-Thomsen and Hansen 2011).

Since 2010, the strong tradition of basing patient involvement on scientific analysis of patient-related aspects through a review of existing and primary research has continued. Specific attention has been given to including researchers with specific competences within social aspects and experience with qualitative research methods in the production of HTA targeted at improving the analysis of patient aspects.

22.6 Future Plans to Develop Patient Involvement

Even though patient aspects have been an integral part of the Danish HTA tradition, the generation of evidence for patients' perspectives can be strengthened. Patient involvement should be continuously discussed, reflected upon and developed. Furthermore, research must also be developed in correspondence with methodological and theoretical developments in the relevant scientific disciplines.

Specifically considering research, the HTA reports could benefit from involving patients more in the research process. In the case of primary data collection among patients, their involvement could include the development of the interview guide, selection of interview population or feedback on the analysed material. Considering systematic literature reviews, patient representatives could advise on the scope and missing issues. Patient participation in the research process could be developed in a format where the patients are not seen as stakeholders, but as 'partners' with a specific knowledge of patient aspects and with the responsibility for safeguarding the inclusion of broader patient perspectives in HTA (Chap. 8). However, it is important to ensure that decisions about research are based on clear and explicit reasoning and reported in a transparent manner.

Even though the institutionalisation of HTA in Denmark is currently weaker than when the responsibility for HTA was national, the regional activities co-ordinated by DEFACTUM still build on the Danish HTA model and include a strong commitment to solid research of patient aspects. In recent years, fewer HTAs have been performed in Denmark. However, this has not led to a reduction in the analysis of patient aspects. This may be because the national and regional healthcare plans emphasise patient-focused care. In addition, the general research skills are used in many broader health service research projects and can thus be maintained.

References

Bennetsen BM, Ehlers L, Overvad K, Groth Jensen L, Løvschal C, Andersen S, Viskum Hansen L, Kjølby M. Medicinsk teknologivurdering af screening for abdominalt aortaaneurisme [health technology assessment of screening for abdominal aorta aneurisms]. Aarhus: Center for Public Health; 2008.

Central Denmark Region. Screening–status og perspektiver [Screening–status and perspectives]. Aarhus: Central Denmark Region; 2009.

Danish Centre for Evaluation and Health Technology Assessment. Introduction to mini-HTA—a management and decision support tool for the hospital service. Copenhagen: Danish Institute for Health Technology Assessment; 2005.

Danish Centre for Evaluation and Health Technology Assessment. Introduktion til mini-MTV–et ledelses-og beslutningsstøtteværktøj til kommunerne [introduction to mini-HTA—a management and decision support tool for the municipalities]. Copenhagen: Danish Institute for Health Technology Assessment; 2008.

Danish Centre for Health Technology Assessment. Follow-up in gynaecological cancer patients—a health technology. Copenhagen: National Board of Health, Danish Centre for Health Technology Assessment; 2009.

Danish Institute for Health Technology Assessment. Medicinsk teknologivurdering. Hvorfor? Hvad? Hvornår? Hvordan? [Health technology assessment. Why? What? When? How?]. Copenhagen: Danish Institute for Health Technology Assessment; 2000.

Danish Institute for Health Technology Assessment. Health technology assessment handbook. Copenhagen: Danish Institute for Health Technology Assessment; 2001.

Hansen HP, Lee A, van Randwijk CB. Patient aspects: a review of fifty-eight Danish HTA reports. Int J Technol Assess Health Care. 2011;7:330–6.

Kristensen FB, Sigmund H, editors. Health technology assessment handbook. Copenhagen: Danish Centre for Health Technology Assessment, National Board of Health; 2007.

Lee A, Skött LS, Hansen HP. Organizational and patient-related assessments in HTAs: state of the art. Int J Technol Assess Health Care. 2009;25:530–6.

National Board of Health. Medicinsk teknologivurdering—hvad er det? [Health technology assessment—what is it?]. Copenhagen: National Board of Health; 1984.

National Board of Health. Medicinsk teknologivurdering—hvad er det? [Health technology assessment—What is it?]. Copenhagen: National Board of Health; 1994.

Palmhøj Nielsen C, Lauritsen SW, Kristensen FB, Bistrup ML, Cecchetti A, Turk E. Involving stakeholders and developing a policy for stakeholder involvement in the European network for health technology assessment (EUnetHTA). Int J Technol Assess Health Care. 2009;25(Suppl 2):44–91.

Sigmund H, Kristensen FB. Does health technology assessment benefit health services and politics? The experiences of an established HTA institution: the Danish Centre for Evaluation and HTA. Eur J Health Econ. 2002;3:54–8.

Sigmund H, Kristensen FB. Health technology assessment in Denmark: strategy, implementation, and developments. Int J Technol Assess Health Care. 2009;25(Suppl 1):94–101.

Tjørnhøj-Thomsen T and Hansen HP. Knowledge in health technology assessment: who, what, how? Int J Technol Assess Health Care 2011;27:324–329.

Chapter 23
England

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23.1 Introduction

The National Institute for Health and Care Excellence (NICE) is established in English law to provide national guidance and advice to improve health and social care. From its inception in 1999, NICE has committed to support the participation of patients, carers (informal caregivers) and people who use health and care services in shaping its guidance, of which HTA is one example. NICE has a policy outlining its approach to patient and carer involvement in its decision-making (NICE 2013a). NICE's Public Involvement Programme (NICE 2016a) provides direct support, resources and training to the patients and patient organisations who participate in NICE's work.

This chapter describes NICE's approach to patient participation across all HTA guidance types. This includes submissions from patient organisations, participation in scoping, individuals attending committee meetings as patient experts, public consultation and an appeal process. NICE also has at least two lay members as part of each decision-making committee and their role is explained. The resources NICE provides to support patient and public involvement and participation are also described. These include dedicated public involvement staff, training, templates and numerous written support resources.

23.2 How NICE Supports Patient Involvement

Involving patients and the public is integral to NICE's work. NICE has a dedicated team, the Public Involvement Programme (PIP), to support and advise on patient and public involvement across all its work programmes. The PIP includes four

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members of staff working on HTAs. The PIP helps identify, train and support the lay people and organisations involved in each piece of guidance. Their work is supported by NICE's public involvement policy (NICE 2013a) and its core principles as set out in its Charter (NICE 2013b).

The PIP provides written materials to support patient organisations and individuals to participate (NICE 2016b). The team also evaluates NICE's involvement approaches with a view to improving their quality.

The terminology used within this chapter is consistent with the terminology used throughout the rest of this book. Some of these terms used however are not consistent with those used by NICE within its processes. For example, the term 'patient input' is used in Sect. 23.4.3, whereas NICE would describe this type of information as 'patient evidence'. At NICE, the term 'evidence' is used to cover a wide range of information sources from formal published research (both qualitative and quantitative), expert submissions and commentary, through to individual testimony and experience.

23.2.1 NICE's Approach to Patient Involvement in HTAs

All NICE HTA guidance is developed by independent advisory committees. Patient involvement in these programmes seeks to embed people's unique perspectives on living with and being treated for their illness, condition or disability. The committees make recommendations across a wide range of clinical topics, and each has two lay people as part of their membership. NICE HTAs broadly follow the same underpinning process, all stages of which have opportunities for participation:

- Scoping
- Evidence gathering
- Committee consideration
- Consultation
- Appeal or resolution
- Publication
- Review

23.3 Health Technologies at NICE

NICE's HTA programmes make recommendations about the funding and use of medicines, medical technologies and procedures for England's National Health Service (NHS).

NICE has four HTA guidance programmes:

- Technology appraisals (TA) (NICE 2016c)—medicines with a marketing authorisation (MHRA 2016, EMA 2016)
- Highly specialised technologies (HST) (NICE 2016d)—medicines for very rare diseases

- Medical technologies (NICE 2016e)—new or existing medical devices
- Diagnostics (NICE 2016f)—new diagnostic technologies

An additional programme, interventional procedures (NICE 2016g), looks at the safety and efficacy of new and innovative surgical procedures, but does not consider issues of clinical or cost-effectiveness.

This chapter focuses primarily on medicines' HTAs and highlights the key differences between these and the other HTA programmes. Both NICE's technology appraisal (TA) and highly specialised technology (HST) HTAs broadly follow the same process and timescales, but have different methodologies and decision-making frameworks.

23.4 HTA for Medicines (Technology Appraisals)

Figure 23.1 presents the NICE process for medicines' HTAs and the opportunities for patient participation at each stage.

23.4.1 Identifying Patient Organisations

PIP contacts organisations that have not been involved in a NICE appraisal before, to explain the role of NICE, how the patient organisation can participate and to encourage contributions and offer support as needed. Relevant national patient organisations are identified by the PIP team from the Internet, the Charity Commission[1] and internal databases. Organisations who wish to participate are sent support materials at the relevant stages of the process and offered an introductory meeting and training session.

23.4.2 Scoping

The topic is scoped using the PICO framework (Chap. 1). Patient organisations are invited to comment on a draft scope document and to attend a workshop. Their insights are sought on the following key aspects:

- Outcomes of importance to patients
- Quality of life issues which may not be captured by conventional measures
- Tolerability and acceptability of the new medicine compared to treatment currently available

After the scope is finalised, the Department of Health decides whether to refer the topic to NICE for appraisal.

[1] Provides registry of not for profit organisations with charitable purposes

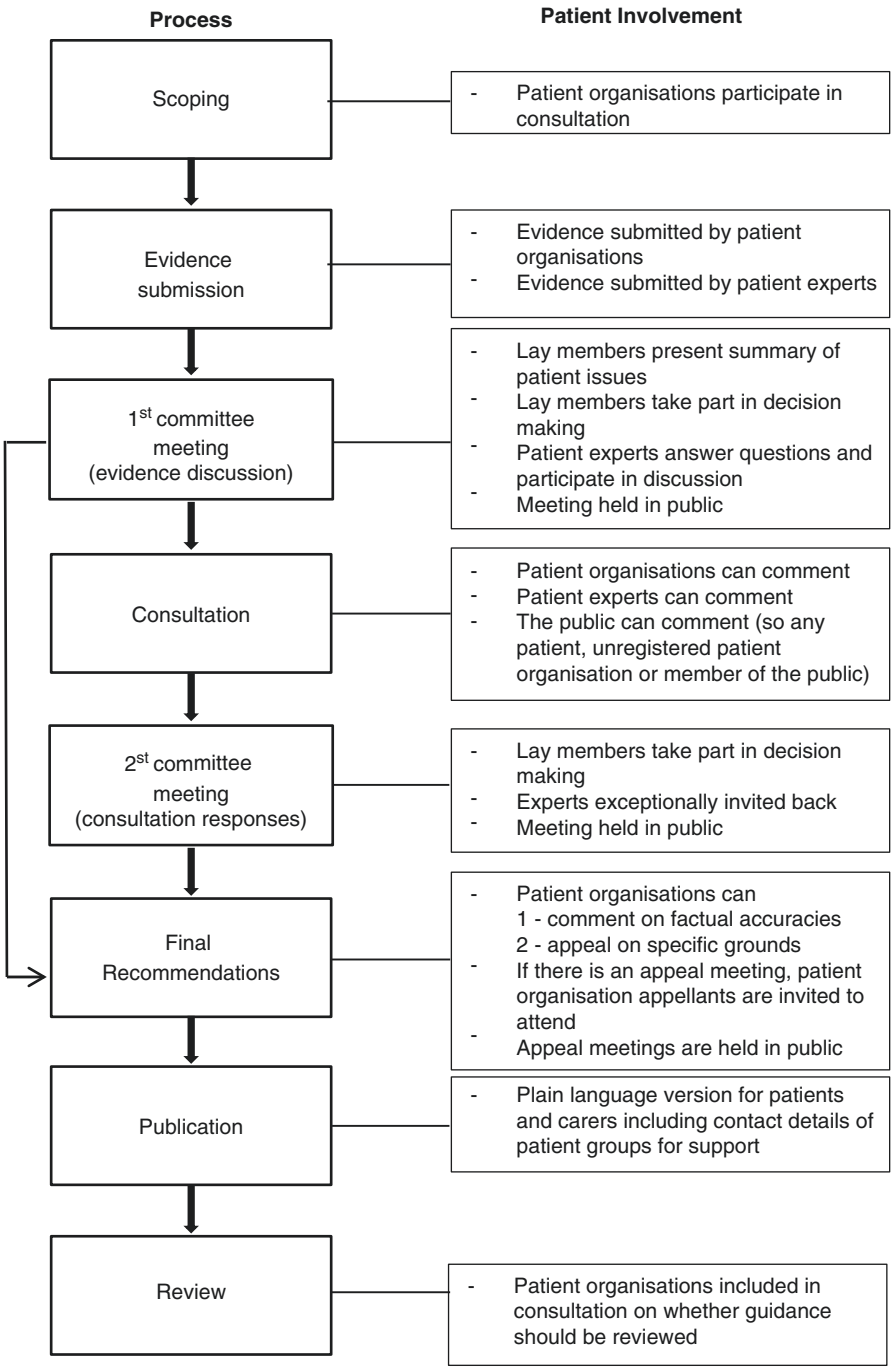

Fig. 23.1 The NICE technology appraisal process—stages of patient involvement

23.4.3 Input from Patients and Patient Organisations

23.4.3.1 Input from Patient Organisations

Patient organisations identified during scoping are invited to submit input for consideration by the committee as part of its decision-making. Each organisation may produce an individual submission, or they may collaborate on a joint submission.

A template structure (NICE 2015a) supports patient organisations in submitting key information (Chap. 6). The evidence submissions from all stakeholders are published as part of the evidence at the consultation stage of the process (see below).

23.4.3.2 Input from Individual Patients

Organisations are asked to nominate people to attend the committee meeting to give testimony and to answer questions from a patient or clinical perspective (NICE 2015b). These 'experts' are also invited to provide a personal written statement (using a template) but do not take part in the committee's decision-making processes. These statements are published together with the other submitted evidence.

Experts attend the committee meeting as individuals rather than as representatives of any organisation. The committee chair selects two patient experts from the nominations received. Ideally one of the experts will be someone with the condition relevant to the treatment being appraised, preferably someone using the new treatment. They provide an in-depth perspective on their individual experience. The other expert usually works or volunteers for a patient organisation and is able to offer the perspectives of a broad range of patients with the condition and their carers.

The patient experts give the committee a unique insight into what it is like to live with a condition and its impact on their life, their family and their ability to work. In addition, they provide insight to the benefits, risks, tolerability, side effects and ease of use of the medicine. The outcomes patients consider to be important may also differ from the clinical outcomes measured in the clinical trials and those incorporated into the cost-effectiveness evidence.

Patient experts are offered a payment for their attendance, as well as reimbursement of their expenses.

23.5 Support for the Patient Experts

Patient experts can find committee meetings daunting due to issues such as the:

- Number of people in the room
- Use of technical terms
- Extensive discussion about clinical and cost-effectiveness compared with discussion of patients' issues

To ameliorate this NICE's PIP has written a guide for patient experts (NICE 2015b) which explains what happens before, during and after a committee meeting, and outlines the experts' role. Patient experts can speak to someone from the PIP to ask any questions they may have and clarify what will happen at the meeting. A member of the PIP meets the patient experts before the meeting starts, and provides support as needed. The committee chair and the lay members play an important role in ensuring the patient experts feel relaxed and confident about participating in the meeting. To support this, one of the lay members will usually sit next to the patient experts during the meeting.

23.5.1 Committee Considerations

NICE committee meetings are divided into two parts: public (part 1) and confidential (part 2). The patient experts, clinical experts and the pharmaceutical company's representatives are invited to attend the first session. This session is also open to members of the public (NICE 2016h) and discusses the clinical, cost-effectiveness and patient issues in detail.

Following 'part 1', only the committee members and NICE staff remain in the meeting. Confidential information (academic or commercial) is discussed, and the initial recommendations are drafted.

Examples of Patient Organisation Submissions

Ulcerative Colitis (vedolizumab)

Patient input informed the committee that the condition meant that patients are often housebound or hospitalised and unable to work or study. It stated that the population is often teenagers or young adults, whose quality of life is affected by the debilitating nature of the condition; they are unable to study or socialise and it also reduces their possibility of meeting a partner. The submission explained why surgery is an inappropriate comparator for young adults; surgery is irreversible, with risks, and has life-long effects, including on fertility. The committee was told that the treatment being considered gave them complete remission and thus 'their life back'.

Ankylosing Spondylitis and Non-Radiographic Axial Spondyloarthritis (TNF-alpha inhibitors)

A patient organisation ran a survey to understand the impact of these conditions on quality of life for people affected. There were 608 responses, which were used to inform the patient organisation's submission. They highlighted the effects on ability to work and on personal life; one-third gave up work before normal retirement age, many patients never married, women were less likely to have children and those who did marry were more likely to divorce.

23.5.1.1 The Role of the Committee Lay Member

Each committee has two lay members with equal status to the other members. The lay members do not 'represent' any particular group or constituency of patients, but bring a broad patient perspective to the committee's decision-making. Although all committee members need to consider many aspects of all decisions, the lay members ensure that issues raised by patients are heard and reflected in the committee's decision-making. For each appraisal, one of the lay members takes 'lead' responsibility, summarising and presenting the relevant patient evidence. Other committee members act as equivalent leads on the clinical and economic evidence.

23.5.1.2 Considering the Evidence

The committee discusses all the available evidence in relation to the medicine under consideration. This includes formal published research (both qualitative and quantitative), economic modelling, expert submissions and commentary, and individual testimony and experience. The committee asks the experts and the pharmaceutical company for their insights and to clarify any issues of uncertainty. The experts are not asked to make a presentation, but can participate in the discussions and answer the committee's questions. Patient experts are always asked if they have anything further to say before they leave the meeting, to ensure that issues important to them are not overlooked.

23.5.2 Consulting on Draft Recommendations

Stakeholders and experts are sent to the committee's provisional recommendations in confidence and have 4 weeks to comment. One week later the draft recommendations are published on the NICE website for a 3-week public consultation period to seek views on whether the draft recommendations are an appropriate interpretation of the evidence considered.

Patient organisations are encouraged to comment on the extent to which the draft recommendations have taken account of the evidence from patients' perspectives. Where there is a lack of evidence or clarity, patient organisations sometimes run surveys with their members during the consultation period to inform any response.

Even where a patient organisation agrees with the draft recommendations, it is important for them to respond to consultations; otherwise NICE only hears from those who disagree.

Example of a Patient Group Response to a Consultation
Ankylosing Spondylitis and Non-Radiographic Axial Spondyloarthritis (TNF-alpha inhibitors)

The patient organisation agreed with most of the committee's recommendations with two exceptions. Firstly, that infliximab was not recommended and, secondly, that a second or subsequent anti-TNF for people whose disease has not responded to treatment with the first anti-TNF, or those who had an initial response which was then lost, was also not recommended. To inform their consultation reply, they ran a survey, gaining 858 responses in 8 days, and the results were submitted to the committee. The recommendations were subsequently amended; firstly, the committee stated that infliximab might benefit people with memory problems, learning disabilities, dexterity problems or a fear of needles and, secondly, that there was also anecdotal evidence suggesting that a second or third TNF-alpha inhibitor can be clinically effective if the first has failed.

23.5.2.1 Considering the Consultation Responses

The committee meets for a second time to discuss the comments received during the consultation. Patient and clinical experts are not normally invited to this meeting; however, if significant new evidence has been submitted, or the committee has questions for the experts, they can be invited to attend.

23.5.2.2 Final Recommendations

Following the second meeting, the final recommendations are drafted and sent in confidence to the stakeholders and experts. This information also includes:

- Discussion of how the committee considered information provided by patient organisations and the patient experts
- Any comments received during the consultation period
- NICE's responses to these comments

The final recommendations are published on the NICE website 1 week later.

23.5.3 Appeal

Stakeholder organisations can comment on factual inaccuracies in the final recommendations and have the opportunity to lodge a formal legal appeal (NICE 2016i) on one or both of the following grounds:

- That whilst developing the recommendations NICE has failed to act fairly or that NICE it has exceeded its powers
- The recommendations are unreasonable in the light of the evidence submitted

Appeals are heard in public in front of a panel (NICE 2014a) comprising five members including a lay person. The appeal panel cannot change the recommendations. However, if any of the appeal grounds are upheld, then the appraisal will return to the relevant stage in the development process (NICE 2014b).

23.5.4 Publication

If no appeal is received, or if the grounds for appeal are not upheld, the recommendations are published on the NICE website as formal guidance to the NHS. The underpinning evidence and submissions obtained throughout the process are published alongside the guidance (HTA report).

A plain language version of each appraisal is also published. These include information on:

- What the guidance means for patients
- A simple explanation of why NICE made the recommendation
- A link to a website called NHS Choices (NHS Choices 2016) for more information about the condition
- Contact information for relevant patient organisations who can provide more information and support

23.5.5 Review

Relevant clinical and cost-effectiveness evidence is normally reviewed between 2 and 4 years after guidance publication (NICE 2014c). NICE consults with patient organisations and other stakeholders on whether the evidence has changed significantly enough for recommendations to be updated.

23.5.6 Highly Specialised Technologies

NICE's evaluations of medicines for very rare conditions are known as highly specialised technology guidance (HST). HSTs use the same general process for participation as described above for TAs, however, the methodology is different to take account of the:

- Very small population
- Higher costs of the technology
- Narrow evidence base and its consequent uncertainties

The committee considers several issues including: the nature of the condition, impact of the new technology, cost to the NHS and personal social services, value for money and impact of the technology beyond direct health benefits.

23.5.6.1 Patient Participation in HSTs

Patient organisations are involved in the scoping process. Patient organisations and patient experts are invited to submit evidence to the committee. As there is often less published evidence on clinical and cost-effectiveness, the committee values highly the input submitted from the patient organisations and experts. This fills gaps in the published research and helps the committee understand the disease and the outcomes of the treatment for patients.

The process is largely the same as TAs; however, unlike TAs, the patient experts are routinely invited to attend both committee meetings: the first where the evidence is considered and the second where consultation responses are discussed.

23.6 HTAs for Technologies Other Than Medicines

23.6.1 Medical and Diagnostic Technology Topic Selection

For a medical or diagnostic technology to be considered by NICE, it must have[2] a CE (Conformité Européene) mark. This confirms the technology has demonstrated conformity with the EU regulations for medical devices, demonstrating its quality, safety and performance. Unlike medicines regulation, clinical studies are not required for all medical devices to demonstrate 'performance'.

Medical and diagnostic technology topics are usually formally notified to NICE by the company who manufactures or distributes the technology. The notification (NICE 2016j) outlines the case for the National Health Service (NHS) to adopt the technology including:

- A description of the patient and healthcare benefits offered by the technology
- The place of the technology in a care pathway
- The other technologies it would either replace or be used alongside

23.6.1.1 Patient Participation in Selecting Medical or Diagnostic Technology Topics

Due to the scarcity of published evidence, input from patient organisations is critical at a very early stage in highlighting information frequently not found in the published evidence such as:

[2] Or be expected to have by the time the guidance is published

- Unmet patient need
- The acceptability of existing or new technologies
- Quality of life benefits of a new technology
- Issues around usability for technologies that may be used by patients themselves

Before a topic is selected, relevant patient organisations are asked to complete a questionnaire to help fill these evidence gaps. Questionnaire responses are considered, alongside other evidence, by NICE's Medical Technology Advisory Committee which decides whether the technology is suitable for guidance development.

23.6.2 Medical Technologies Guidance

NICE medical technology evaluations consider a single technology and its:

- Benefit to patients over and above existing technologies
- Benefit to the healthcare system—for example, potential reduction in burden on staff, patients or healthcare resources

The NICE process for evaluating medical technologies is broadly similar to its process for appraising medicines and has equivalent committee and patient participation mechanisms. The one notable exception is in relation to how topics are selected for evaluation (see above).

Minor differences include a shorter scoping process—replacing the scope consultation with a 5-day fact checking exercise—and a resolution process in place of a formal appeal mechanism. Patient experts are included in the process where the patients are the users or operators of the technology or where patient outcomes and preferences are particularly important.

Medical technology evaluations look at the cost impact (rather than cost-effectiveness) of adopting a new technology. A new technology may be more expensive than an existing one, but it may require fewer staff to operate or mean that a patient needs to be in hospital for less time. As such it might have the potential to reduce overall expenditure.

23.6.3 Diagnostics Guidance

NICE's assessment of diagnostic technologies considers one or more technologies, usually for use in a single care pathway.[3] At the time of writing, there is no formal mechanism for patient organisations to submit input for consideration by the

[3] Although the facility exists to consider a variety of applications for a diagnostic technology across several conditions

committee; however, work is ongoing to address this. As with medical technologies guidance, a resolution process takes the place of a formal appeal process.

23.6.3.1 Patient and Carer Specialist Committee Members

The participation of patients and carers in diagnostics assessment differs from that of the technology appraisal and medical technology processes. The limitations of the evidence-based and frequent uncertainty about where in the care pathway a diagnostic technology should be used means that expert input is required from the beginning and throughout the development process.

Patients and clinicians with expertise related to an individual technology are recruited as full voting members of the Diagnostics Advisory Committee, alongside the core members who consider each technology. Known as 'specialist committee members', these experts are recruited at the beginning of the development process. Specialist committee members take part in meetings contributing their knowledge, expertise and experiences and also taking part in the formal committee decision-making.

Whilst only one patient or carer specialist committee member is appointed to the committee for each particular topic, all applicants for the specialist committee member role, along with relevant patient organisations, are invited to a workshop at the start of the process. This is to ensure that as much patient expertise as possible is available to NICE when discussing the scope of the guidance.

In many cases a diagnostic technology is not a treatment in itself—the benefit of using the technology may only become apparent at a much later stage in the patient's care. By inviting patients, carers and patient organisations to take part in the initial workshop, NICE can ensure that the resulting scope best reflects the needs and preferences of the people who may benefit from the technology.

23.7 Evaluating Patient Participation in HTAs

In 2012 a formal evaluation was conducted of the experiences of the patient experts who had participated in NICE's technology appraisals process. The evaluation explored the impact they felt they made to NICE's decision-making (NICE 2014d). This revealed a number of useful practical suggestions to improve our approach such as: chairs formally introducing themselves to the experts; chairs introducing the role of the experts for the benefit of the public gallery; and clarifying the difference between the lay members and the patient experts. These suggestions have now been incorporated as part of the action plan (NICE 2015c). In addition, patient experts are now routinely asked to record their experiences of participation to support continuous quality improvement. This is part of a wider approach to systematically gather experience and impact data from all lay people working with NICE (NICE 2015d). As part of this approach, all lay members leaving any of the HTA

committees complete an exit questionnaire which feed into quarterly reports with recommendations for improvements.

23.8 Options for Future Development

At the time of writing, the patient involvement team is undertaking an evidence-based review to identify a clear strategic vision for NICE's future public involvement activities and to identify ways of putting this into practice. Early findings indicate a need to review our approach to evidence submissions and the patient expert role. We have since gone out for consultation so this either needs to be deleted or replaced with 'The proposals for change have now been subject to public consultation'. NICE will continue to engage with patient organisations and individuals on developing and evaluating meaningful patient participation, so that HTA guidance includes their preferences and priorities.

References

EMA. Homepage. 2016. http://www.ema.europa.eu/ema/. Accessed 31 Mar 2016.

MHRA—Medicines and Healthcare Products Regulatory Agency. Homepage. 2016. https://www.gov.uk/government/organisations/medicines-and-healthcare-products-regulatory-agency. Accessed 31 Mar 2016.

NICE. Patient and public involvement policy. 2013a. http://www.nice.org.uk/about/nice-communities/public-involvement/patient-and-public-involvement-policy. Accessed 31 Mar 2016.

NICE. NICE charter. 2013b. https://www.nice.org.uk/Media/Default/About/Who-we-are/NICE_Charter.pdf. Accessed 31 Mar 2016.

NICE. The appeal panel. 2014a. https://www.nice.org.uk/article/pmg18/chapter/2-The-Appeal-Panel. Accessed 31 Mar 2016.

NICE. After the appeal. 2014b. https://www.nice.org.uk/article/pmg18/chapter/10-After-the-appeal. Accessed 31 Mar 2016.

NICE. Reviews. 2014c. http://www.nice.org.uk/article/pmg19/chapter/6-Reviews. Accessed 31 Mar 2016.

NICE. Technology appraisals patient expert survey 2012 report. 2014d. http://www.nice.org.uk/media/default/About/NICE-Communities/Public-involvement/Public-involvement-programme/Patient-expert-TA-report-final-1.pdf. Accessed 31 Mar 2016.

NICE. Single technology appraisal patient organisation submission template. 2015a. https://www.nice.org.uk/Media/Default/About/NICE-Communities/Public-involvement/Developing-NICE-guidance/Patient-Organisation-STA-Template.docm. Accessed 31 Mar 2016.

NICE. Nominating patient experts. 2015b. http://www.nice.org.uk/Media/Default/About/NICE-Communities/Public-involvement/Developing-NICE-guidance/Nominating-Patient-Experts.pdf. Accessed 31 Mar 2016.

NICE. Public involvement survey action plan. 2015c. http://www.nice.org.uk/Media/Default/About/NICE-Communities/Public-involvement/Public-involvement-programme/Public-involvement-plan-survey-action-plan-4.pdf. Accessed 31 Mar 2016.

NICE. Public involvement programme annual report 2014, 'feedback from exit survey' p. 20. 2015d. http://www.nice.org.uk/media/default/About/NICE-Communities/Public-involvement/Public-involvement-programme/PIP-annual-report-apr-2015.pdf. Accessed 31 Mar 2016.

NICE. Public involvement programme. 2016a. http://www.nice.org.uk/about/nice-communities/public-involvement/public-involvement-programme. Accessed 31 Mar 2016.
NICE. Help us develop NICE guidance. 2016b. https://www.nice.org.uk/about/nice-communities/public-involvement/develop-nice-guidance. Accessed 31 Mar 2016.
NICE. NICE technology appraisal guidance. 2016c. http://www.nice.org.uk/About/What-we-do/Our-Programmes/NICE-guidance/NICE-technology-appraisal-guidance. Accessed 31 Mar 2016.
NICE. NICE highly specialised technology guidance. 2016d. http://www.nice.org.uk/About/What-we-do/Our-Programmes/NICE-guidance/NICE-highly-specialised-technologies-guidance. Accessed 31 Mar 2016.
NICE. NICE medical technologies guidance. 2016e. http://www.nice.org.uk/About/What-we-do/Our-Programmes/NICE-guidance/NICE-medical-technologies-guidance. Accessed 31 Mar 2016.
NICE. NICE diagnostics guidance. 2016f. http://www.nice.org.uk/About/What-we-do/Our-Programmes/NICE-guidance/NICE-diagnostics-guidance. Accessed 31 Mar 2016.
NICE. NICE interventional procedures guidance. 2016g. http://www.nice.org.uk/About/What-we-do/Our-Programmes/NICE-guidance/NICE-interventional-procedures-guidance. Accessed 31 Mar 2016.
NICE. Meetings in public landing page. 2016h. https://www.nice.org.uk/Get-Involved/Meetings-in-public. Accessed 31 Mar 2016.
NICE. Technology appraisal and highly specialised technologies appeals. 2016i. https://www.nice.org.uk/about/what-we-do/our-programmes/nice-guidance/nice-technology-appraisal-guidance/technology-appraisal-and-highly-specialised-technologies-appeals. Accessed 31 Mar 2016.
NICE. Medical technologies evaluation programme notification form. 2016j. http://www.nice.org.uk/Media/Default/About/what-we-do/NICE-guidance/NICE-medical-technologies/MTEP-notification-form.docx. Accessed 31 Mar 2016.
NHS Choices. Homepage. 2016. http://www.nhs.uk. Accessed 31 Mar 2016.

Chapter 24
EUnetHTA: Patients' Perspectives in the HTA Core Model®

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24.1 Introduction

EUnetHTA has developed the HTA Core Model® as a methodological framework for collaborative production and sharing of HTA information (Lampe et al. 2009; Pasternack et al. 2009). In this chapter, the construct of the HTA Core Model® version 3.0 is described. The HTA Core Model® provides a construction for HTA that gives a comprehensive assessment of nine domains, each of which represents an aspect of the use of a given health technology (Pasternack et al. 2009). The processes and inputs that shaped the creation of the Patients' and Social Aspects domain are explored. A description of the final content and scope of the Patients' and Social Aspects domain and its relation to other HTA Core Model® domains is provided, and ways to strengthen research on patients' perspectives in HTA are discussed. The HTA Core Model® stresses the importance of a multidisciplinary design when conducting HTAs to provide a sufficient and solid political decision aid. However, challenges remain in using the Patients' and Social Aspects domain. This is discussed in the concluding section in suggestions on how patients' perspectives may be integrated into future EUnetHTA collaboration.

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24.2 EUnetHTA and the HTA Core Model®

EUnetHTA is a European collaboration of HTA bodies that was established in 2006 at the request of the EU. Each EU member state appoints the HTA bodies that participate. The primary goal of the collaboration or network is to support cooperation between European HTA bodies and avoid duplication of work (Kristensen et al. 2009). The purpose is to produce benefit at a European, national and regional level.

EUnetHTA wishes to develop and support the production of reliable, timely, transparent and transferable HTA information by making available different tools (EUnetHTA 2010). Every EUnetHTA product undergoes a process of public consultation during the drafting process. The HTA Core Model® has been developed over the past decade as a result of feedback on its use by EUnetHTA members and by public consultation. The HTA Core Model® v3.0 (hereafter called the HTA Core Model®) that was issued in 2016 is briefly described in this section. The HTA Core Model® consists of three main components (EUnetHTA 2016b):

- *HTA ontology*, which contains an extensive list of generic questions (*issues)* that can be asked in HTA
- *Methodological guidance*, which helps the researcher find answers to the questions defined by the ontology
- *The common reporting structure*, which provides a standard format for the output of HTA projects

The HTA Core Model® organises the information within an HTA by dividing it into nine *domains* to ensure a broad framing of HTA projects and not just a focus on issues limited to clinical effectiveness and economics. The nine domains are outlined below:

- Health Problems and Current Use of Technology
- Description and Technical Characteristics of Technology
- Safety
- Clinical Effectiveness
- Cost and Economic Evaluation
- Ethical Analysis
- Organisational Aspects
- Patients' and Social Aspects
- Legal Aspects

Each domain is divided into several *topics* (overall subjects like patients' perspectives), and each topic is further divided into several *issues* (specific generic questions to consider the relevance of when conducting an HTA). The combination of domains, topics and issues defines an assessment element within the HTA Core Model®.

Within the HTA Core Model® framework, all nine domains are considered equally important in conducting the HTA and in the subsequent decision process. The list of generic questions to consider consists of questions termed as core

Table 24.1 The HTA Core Model® matrix

Core matrix		Importance		
		1 Optional	*2 Important*	*3 Critical*
Transferability	*3 Complete*	Not core	Core	Core
	2 Partially	Not core	Core	Core
	1 None	Not core	Not core	Core

questions and noncore questions alike. These categories are built on a matrix in consideration of the importance and transferability of each specific question. Transferability is low for information highly specific to a particular context and is most likely not as useful in other settings. Importance is concerned with whether the question contains significant information from the viewpoint of HTA in general. The HTA Core Model® matrix is shown in Table 24.1.

Following this logic, all nine domains have questions belonging to and not belonging to the core. Despite not belonging to the core, questions can often be of significant interest to HTA projects and sometimes of critical importance in a local setting. It is an attempt to guide the user of the model as opposed to a deterministic way of defining the questions in the model.

To further emphasise the equal importance of each domain and the relational nature of many aspects in the HTA process, the HTA Core Model® defines relations between topics and issues across different domains. The model defines two different kinds of relations:

- *Sequential relations*, where it becomes easier to answer question B if the answer to question A is provided
- *Content relations*, which indicate associations between questions that deal with partially similar themes

For a full description of the HTA Core Model®, see EUnetHTA (2016a).

24.3 Aim of the Patients' and Social Aspects Domain

The aim of involving Patients' and Social Aspects in HTAs is to understand how health technologies are used, perceived and shaped in everyday life among patients. In recent years, an ontological shift in perceiving health technologies has taken place. This implies a change from seeing health technologies as more or less separated from their surroundings and acting independently from their human and non-human contexts to a multifaceted way of understanding health technologies (Lehoux 2006; Facey et al. 2010; Koivisto et al. 2010; Hansen and Lee 2011). This way of addressing health technologies is inspired by actor-network theory (Latour 1987), among others.

In such a perspective, patients' aspects indicate that a health technology does not produce effects in isolation from the patients who are using the technology. Rather,

the patients are perceived as co-producers. This happens when patients use, act and respond to the technology. When perceiving health technologies in a co-productive way, the central research question would be: *How do patients act with a technology? What resources does it require?* (Koivisto et al. 2010).

This development of patients' perspectives in HTA is in line with an ontological shift in healthcare from a biomedical model to a biopsychosocial model, which is a social medical model developed by George Engel (1977). Biomedical models attribute diseases mainly to biological factors and somatic abnormalities and characterise health by the absence of disease. In contrast, the biopsychosocial model understands disease and health as an interaction of individual biological, psychological and social factors.

Following such developments, versions 1 and 2 of the HTA Core Model® included the Social Aspects domain, and following consultation this was modified in version 3.0 of the model to be the Patients' and Social Aspects domain (2016). This altered the core questions to be more focused on issues relevant to patients as outlined in the HTAi Interest Group submission templates (HTAi 2014) and updated the methodological guidance to include new sources for literature searching. In the following sections, the form and content of the domain will be further expanded.

24.4 Content of Patients' and Social Aspects Domain

The Patients' and Social Aspects domain v3.0 concerns 'topics' and 'issues' relevant both to patients who receive and use health technologies and health services and to caregivers from the social network who provide care for patients. The domain seeks to identify evidence about experiences, expectations, valuations and opinions related to the health technology among patients and caregivers, their experiences of living with the condition being studied and the consequences (e.g. effect and efficiency) for everyday life when using the technology.

The interaction between the use of the technology, a person's health and other personal and environmental factors is a central focus of the domain. A health technology is implemented in a hospital or a primary care setting, but its use may have implications for patients beyond the original setting of the technology to the homes and everyday lives of the patients. Patients are also members of families, communities and societies, and thus both individual illnesses and treatments affect significant others (spouses, parents, children, friends, etc.), who take part in shaping the use of the technology through their support or restrictions.

It is also recognised that there are underlying communication issues, so these are also studied in the domain. Examples include communication between health personnel and the patient about the use and implication of the technology, communication about the meaning of the results of diagnostic or genetic tests for a wider diagnosis pathway and communication about self-administered devices, all of which are important for the use of and decisions about the technology.

Figure 24.1 shows the above-described themes that contribute to the Patients' and Social Aspects domain. The figure draws on inspiration from an analytical

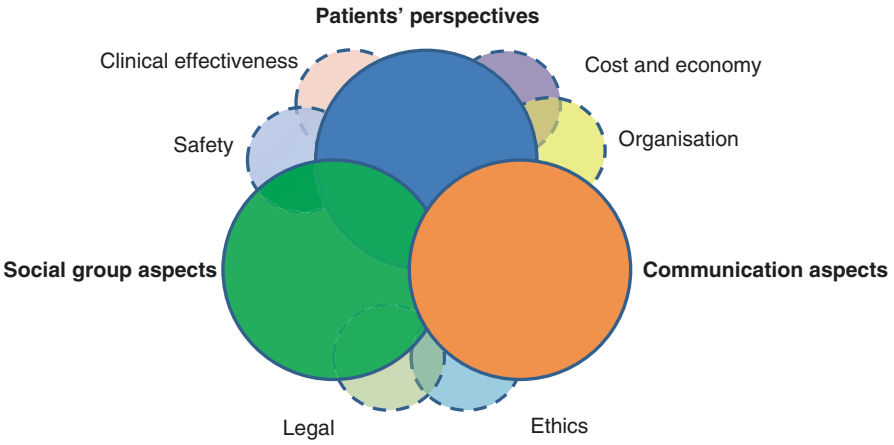

Fig. 24.1 Themes of the Patients' and Social Aspects domain and relationship to other domains of the HTA Core Model® (HTA Core Model version 3.0 2016)

model developed by Hansen (Hansen 2007). This model shows how subjects, which have been considered relevant in previous HTA reports regarding patients' perspectives, are empirically related and that it is only analytically possible to focus on single aspects at a time. Figure 24.1 is a further development of the model, illustrating the themes of Patients' and Social Aspects domain in terms of the topics of patients, social groups and communication, as well as its relation to the other domains of the HTA Core Model®.

The Patients' and Social Aspects domain contains eight issues, which in turn are related to the three topics of the patients' perspectives, social group aspects and communication aspects, as shown in Fig. 24.1. Table 24.2 shows the topics and issues of the final version of the domain and how it has changed from the previous version.

24.4.1 Methods

The HTA Core Model® (EUnetHTA 2016) provides a handbook with detailed guidance on conducting research into patients' perspectives as part of a HTA Core Model but presents approaches that are relevant for anyone doing an HTA.

Similar to any HTA work, methods for answering issues relating to patients' perspectives should start by conducting a literature search to examine whether it is possible to answer the chosen research questions by a synthesis or a meta-analysis of existing studies (Chap. 15). If this is not possible or if it provides insufficient evidence, primary studies should be conducted. Evidence on patients' perspectives can be derived from both qualitative and quantitative approaches (Part II). Depending on the type of research questions, a multi-method research design could also be used. Finally, patient participation in HTA conduct should be considered but is often ignored (Chaps. 5, 8 and 13).

Table 24.2 Topics and issues in Social Aspects domain v2.0 and in Patients' and Social Aspects domain v3.0

Social Aspects domain v2.0		Patients' and Social Aspects domain v3.0	
Topic	Issue	Topic	Issue
Individual	What kind of changes do patients or citizens expect?	Patients' perspectives	What are the experiences of living with the condition?
	Who are the important others that may be affected, in addition to the individual using the technology?		What expectations and wishes do patients have for the technology and what do they expect to gain from the technology?
			How do patients perceive the technology under assessment?
	What kind of changes may the use of the technology generate in the individual's role in the major life areas?		What is the burden on caregivers?
	How do patients, citizens and the important others using the technology react and act upon the technology?		
	Are there factors that could prevent a group or person from gaining access to the technology?		
	What is the socio-economic impact of the technology to the patient and his/her important others?		
Major life areas	What kinds of reactions and consequences can the introduction of the technology cause at the overall societal level?	Social group aspects	Are there groups of patients who currently don't have good access to available therapies?
	Which social areas does the use of the technology influence?		Are there factors that could prevent a group from gaining access to the technology?
	What influences patients' or citizens' decisions to use the technology?		
	How does the technology affect inequalities in health?		

(continued)

Table 24.2 (continued)

Social Aspects domain v2.0		Patients' and Social Aspects domain v3.0	
Information exchange	What is the knowledge and understanding of the technology in patients and citizens?	Communication aspects	How are treatment choices explained to patients?
	What are the social obstacles or prospects in the communication about the technology?		What specific issues may need to be communicated to patients to improve adherence?

24.5 Relation of Patients' and Social Aspects Domain with Other Domains

The ultimate aim of the existence and use of technologies in health services is for patients to benefit. Overall, the full HTA Core Model® describes and analyses the consequences of a given technology for the patients from different perspectives (Fig. 24.1). In this way, the nine domains of the HTA Core Model® ultimately concern the patients. As such, patients' perspectives can be present in several other domains of the HTA Core Model®. This could be the case if patient-related issues are estimated at a societal level. For example, issues related to socio-economic benefits could be covered in the Cost and Economic Evaluation domain, or issues about the provision of healthcare and equitable allocation of resources could be covered in the Organisational Aspects domain. Ethical and/or political topics discussed in the Ethical Analysis or Legal Aspects domains would often be closely related to issues relevant to the Patients' and Social Aspects domain. In addition, patients' perspectives on biological/physical/psychological topics could be connected to the Clinical Effectiveness or Safety domains, for example, individuals may receive various benefits from technologies that can be measured as changes in disease or improved functioning in their everyday lives.

However, these other domains do not specifically address experiences, evaluations, needs and expectations based on patients' personal knowledge and experiences. Furthermore, the focus of other domains may be on specific health issues, whereas the patients and their caregivers are the only ones going through the longitudinal course of illness across healthcare sectors and homes in everyday life. In addition, the methods for collecting evidence can be domain specific and, as such, differ from the Patients' and Social Aspects domain. It is therefore important to specifically address patients' issues as an independent part of a full HTA. It is still essential to stress that coordination is needed across all domains in order to exchange information and avoid overlap when producing an HTA in a Core Model frame. As described in Sect. 24.2, attention should be given to the types of relationships between topics and issues across the domains of a full HTA. Thus, topics and issues could have similar subject matter or substance (content relationship) or they could

form a logical order so the questions should be answered in a sequence because it is easier, or makes sense, to answer question B if the answer to question A is provided first.

24.6 Discussion

The development of the HTA Core Model® stresses that a multidisciplinary research perspective is needed to create a robust basis for political decision processes. However, challenges remain in using the Patients' and Social Aspects domain.

Although the literature on patients' perspectives and methods for obtaining evidence on patient issues are substantial, expert skills in the social sciences are required to conduct this part of an HTA. This seems limited in most HTA bodies (Lehoux and Williams-Jones 2007; Facey et al. 2010).

Furthermore, a major intention of the EUnetHTA collaboration is the transnational use of HTAs, but studies on patients' aspects are challenged by transferability and generalisability. Patients' and Social Aspects of a given health technology are context dependent to a great extent. Issues such as the roles and obligations of patients and caregivers, the social network, cooperation between health professionals and patients, disease perceptions and social consequences of diseases depend heavily on specific social and cultural environments. This might not be different from health economic issues, for example, where societal differences in healthcare sector financing and reimbursement are apparent. However, economic and clinical effectiveness have played a dominant role in HTAs, and therefore, more profound experiences with generalisability and the transnational use of results exist within these domains. So more needs to be done to consider the issues of transferability in relation to patients' aspects. Methodological developments in patient-centred outcome research (PCOR) as shown by PCORI[1] Methodology Standards (PCORI 2016) might be useful to overcome some of the challenges of transferability across nations. PCORI uses patient-centred outcome research to improve methodology of comparative effectiveness research that seeks to give voice to patients and caregivers in health decision-making (Snyder et al. 2013).

Patients' perspectives will be explored as part of the full HTA Core Models® implemented by national and regional HTA bodies in the coming years. However, it is also interesting to consider how patients' perspectives may be integrated into the future EUnetHTA collaboration. At the time of writing, EUnetHTA Joint Action 3 for 2016–2020 is underway. In this new joint action, there is a focus on the production of international, collaborative HTAs. However, it has been decided to focus on the production of Rapid Relative Effectiveness Assessments (REA). This only includes four domains of the HTA Core Model®, namely, *Description and Technical Characteristics of Technology*, *Health Problems and Current Use of Technology*, *Clinical Effectiveness* and *Safety*. The remaining five domains, including the

[1] Patient-Centered Outcomes Research Institute

Patients' and Social Aspects domain, are assessed by a checklist and , among other things, will guide how patient groups are involved. This appears to indicate that patients' perspectives are given a fairly low priority in future EUnetHTA work. Furthermore, as limited time and resources appear to constitute hurdles to involving patients, this is also likely to limit patient involvement in EUnetHTA. As Hailey and Nordwall (2006) point out, decision-makers do not seem willing to fully commit resources to obtaining evidence on patients' perspectives. Furthermore, Carman et al. (2015) (Chap. 6) indicated the need to further develop methods and focus on involving patients as stakeholders in informing HTAs. As the HTA Core Model® is freely available for anyone to use and provides a useful methodological guide for all stakeholders, it could help address this need.

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References

Carman KL, Mallery C, Maurer M, et al. Effectiveness of public deliberation methods for gathering input on issues in healthcare: results from a randomized trial. Soc Sci Med. 2015;133:11–20.

Engel G. The need for a new medical model: A challenge for biomedicine. Science. 1977;196:129–36.

EUnetHTA. About Us. 2010. Website: http://www.eunethta.eu Accessed 5. Aug 2016.

EUnetHTA. HTA Core Model® Online. 2016a. http://www.htacoremodel.info Accessed 5 Aug 2016.

EUnetHTA. HTA Core Model Version 3.0: 2016b. Website: https://meka.thl.fi/htacore/model/HTACoreModel3.0.pdf.

Facey K, Boivin A, Gracia J, et al. Patients' perspectives in health technology assessment:a route to robust evidence and fair deliberation. Int J Technol Assess Health Care. 2010;26:334–40.

Hailey D, Nordwall M. Survey on the involvement of consumers in health technology assessment programmes. Int J Technol Assess Health Care. 2006;22:497–9.

Hansen HP. The patient. In: Kristensen FB, Sigmund H, editors. Health technology assessment handbook. Danish Center for HTA. National Board of Health: Copenhagen; 2007.

Hansen HP, Lee A. Patient aspects and involvement in HTA: An academic perspective. Pharma Policy Law. 2011;13:123–8.

HTAi. 2014. http://www.htai.org/interest-groups/patient-and-citizen-involvement/resources/for-hta-agencies-and-policy-makers.html Accessed 5 Aug 2016.

Koivisto J, Anttila H, Ikonen T, et al. A systematic model for evaluating the patient aspects of health technologies. Evid Policy. 2010;6:33–49.

Kristensen FB, Mäkelä M, Allgurin NS, et al. European network for health technology assessment, EUnetHTA: planning, development and implementation of a sustainable European network for health technology assessment. Int J Technol Assess Health Care. 2009;25(Suppl 2):107–16.

Lampe K, Mäkelä M, Garrido MV, et al. The HTA Core Model: a novel method for producing and reporting technology assessments. Int J Technol Assess Health Care. 2009;25(Suppl 2):9–20.

Latour B. Science in action: how to follow scientists and engineers through society. Cambridge: Harvard University Press; 1987.

Lehoux P. The problem of health technology. Routledge, NY: Policy implications for modern health care systems; 2006.
Lehoux P, Williams-Jones B. Mapping the integration of social and ethical issues in health technology assessment. Int J Technol Assess Health Care. 2007;23:9–16.
Pasternack I, Anttila H, Mäkelä M, et al. Testing the HTA core model: Experiences from two pilot projects. Int J Technol Assess Health Care. 2009;25(Suppl 2):21–7.
Patients' and Social Aspects Domain: pp. 346–71. Accessed 5 Aug 2016.
PCORI. Patient-centred comparative effectiveness research. 2016. http://www.pcori.org. Accessed 5 Aug 2016.
Snyder CF, Jensen RE, Segal JB, et al. Patient-reported outcomes (PROs): Putting the patient perspective in patient-centred outcomes research. Med Care. 2013;51:73–9.

Chapter 25
Germany

Sabine Haefner and Martin Danner

25.1 Introduction

This chapter presents how Germany supports patient involvement in HTA and decision-making processes in the Federal Joint Committee (Gemeinsamer Bundesausschuss, G-BA) and the Institute for Quality and Efficiency in Healthcare (Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen, IQWiG), which drafts HTA reports commissioned by the G-BA. It outlines processes for incorporating individual and collective patient experiences through participation processes and research, and it shows how Germany has set a legal framework for patient participation. It explains how patient experiences are introduced by patient organisations that have been identified as *relevant organisations* by lawmakers and that have been granted participation rights including means for capacity building. It presents a range of mechanisms for participation according to the mosaic for participation (Chap. 5). In particular, it illustrates how patient organisations use their rights by filing requests for HTAs and decision-making regarding benefit coverage and by identifying patient representatives with different perspectives to take part in assessment and appraisal processes. In conclusion, the chapter reflects on issues of evaluation and future challenges.

Approximately 90% of the people living in Germany are insured under statutory health insurance (SHI) (National Association of Statutory Health Insurance Funds 2016). The SHI is based on the basic principles of solidarity, medical necessity and benefit in kind. Everyone insured under SHI has the same right to necessary medical care regardless of age, disease or the amount of premiums paid.

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25.1.1 Self-Government

Further characteristics are the principles of subsidiarity and *self-government*. The legislators only define the framework for medical care. In many areas, policy making is up to the so-called joint self-government to determine the scope of services provided by SHI funds and to organise healthcare delivery. The joint self-government enables joint decision-making including bodies with representatives from the health insurance funds and care providers (physicians, dentist, physiotherapists and hospitals). They are required by law to provide 'needs-based and equitable care for insured persons according to the current generally accepted state of medical knowledge' (Social Code Book V. Section 12 1992). The 'medical care of insured persons must be adequate, sufficient, and expedient, must not go beyond what is necessary, and must be cost-effective and of high professional quality' (Social Code Book V. Section 12 1992) (Social Code Book V. Section 70 1999). HTAs play a key role in defining these requirements.

25.1.2 Mandate of G-BA and IQWiG

The G-BA is the highest decision-making body of the joint self-government. It is responsible for the assessment, appraisal and decision-making regarding various technologies within SHI, in particular coverage of medical diagnostic and therapeutic methods or pharmaceuticals. Appraisal includes consideration of assessments commissioned to the IQWiG or undertaken within G-BA.[1,2] Moreover, the G-BA develops regulations on organisational and quality aspects of healthcare. G-BA stipulations take the form of directives that are legally binding for all statutory health insurance funds, care providers and insured persons (Fig. 25.1).

25.1.3 Patient Participation

Since 2004, the legislation has supported comprehensive patient participation in the G-BA and the IQWiG. It includes participation of individual patients or their carers (informal caregivers) as well as representatives of patient groups, advocacy groups or consumer organisations. All possible patients' perspectives are covered as described by HTAi (HTAi 2015). The persons participating are chosen and appointed as *knowledgeable persons* (*patient representatives)* by *relevant patient organisations* (Sect. 25.2.2.1) into the bodies of G-BA. The diversity of individual and

[1] The tasks of the G-BA are extensive. For more information on the G-BA, also in English, please visit www.english.g-ba.de/.

[2] For more information on the IQWIG, also in English, please visit www.iqwig.de.

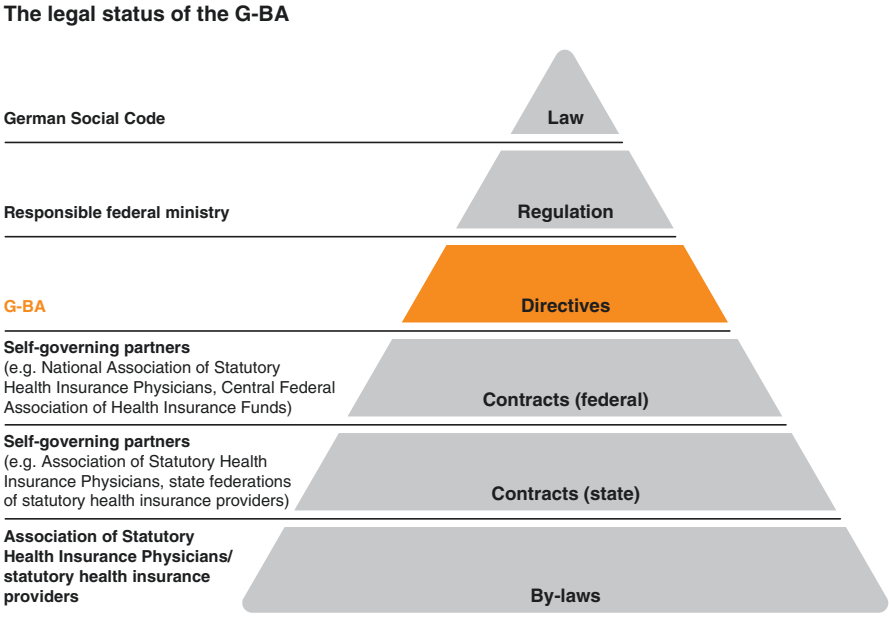

Fig. 25.1 The legal status of the G-BA (G-BA 2013)

collective perspectives during appraisal is intended, and the terms *knowledgeable person* and *patient representative* are used alternatively.

Currently, around 250 *patient representatives* are active in G-BA committees.

25.2 Rationale of Patient Participation in G-BA and IQWiG

25.2.1 Starting Point for Lawmakers

It is no coincidence that patient participation became part of HTA and other decision-making processes of the G-BA and IQWiG.

Improving quality, efficiency and cost-effectiveness The primary aim of a 2003 legislation was to maintain the basic principles of SHI in the context of an ageing society and advancements in medical care, without rationing services. Greater efficiency and better quality in medical care should lead to more cost-effective services overall (SHI Modernisation Act 2003).

The G-BA was established as the decision-making body (thus replacing its predecessor organisations) (Draft SHI Modernisation Act 2003a), and the IQWiG founded as an independent HTA institute (Draft SHI Modernisation Act 2003c).

Self-responsibility and patient sovereignty However, the Modernisation Act also put some services into the category of 'self-responsibility', meaning that the patients

must pay for some services themselves or make co-payments. At the same time, 'patient sovereignty' should be promoted with measures, such as the right to demand an understandable invoice for medical (in-kind) services or to choose cost refund instead of in-kind benefits.

On the macro-level those affected by SHI, the patients, became participants in decision-making processes. This was considered by the lawmakers as the only way more 'self-responsibility' could be asked of them (Draft SHI Modernisation Act 2003b).

25.2.2 The Scope of Patient Participation

The *SHI Modernisation Act* and the *Patient Involvement Act* from 2003 have formed the scope of patient participation in self-government bodies.

25.2.2.1 Relevant Patient Organisations

The Federal Ministry of Health has recognised four *relevant patient organisations* comprising national umbrella organisations and meeting further requirements (Patient Involvement Act 2003).

The four *relevant organisations* are the German Disability Council (Deutscher Behindertenrat 2016), the National Association of Patient Advisory Centres (Bundesarbeitsgemeinschaft der PatientInnenstellen 2016), the German Association of Self-Help Groups (Deutsche Arbeitsgemeinschaft Selbsthilfegruppen 2016) and the Federation of German Consumer Organisations (Verbraucherzentrale Bundesverband 2016). With their member patient organisations, they cover a broad spectrum of diseases and disabilities. This allows for the participation of persons directly concerned, meaning (chronically ill) patients, persons with disabilities and their informal carers. They also include organisations that advise patients. The broad and highly differentiated competence of the various organisations ensures that *patient representatives* can be identified for each specific issue.

The organisations have non-profit status and have to disclose their financing to prove that their work is impartial and independent. *Patient representatives* from member organisations with limited funding from health technology developers can be involved.

25.2.2.2 Participation Rights

The *relevant patient organisations* were granted the statutory rights to take part in (*consultation right*) and to initiate decision-making processes (*right to request a decision*) in the G-BA. The *consultation right* includes the right to be present when a decision is made. They further were given the right to submit *written statements* or take part in *hearings* regarding decisions of other self-government committees

(Social Code Book V. Section 140f 2015d) or the assessments of the IQWiG (Social Code Book V. Section 139a 2015c).

25.2.2.3 Knowledgeable Persons: The Patient Representatives

To exercise their *consultation rights*, the *relevant patient organisations* mutually nominate *knowledgeable persons* (*patient representatives)* with the relevant competence.

A *patient representative* is identified by a *relevant patient organisation* or one of their member organisations. He or she has to be voluntarily or professionally active within a patient organisation. Depending on the subject of consultation, the competence ranges from having individual or collective patient experience within the indication of an assessed technology, to being able to provide a broader knowledge from a consumer perspective. At least half of the *knowledgeable persons* should themselves be directly affected (Social Code Book V. Section 140f 2015d).

The number of appointed persons in the committees, subcommittees and working groups is limited by the number of persons sent by the health insurance funds (Social Code Book V. Section 140f 2015d) (Patient Involvement Act 2003).

25.2.3 Assessment of Rationales

Taking the HTAi values for patient involvement in HTA as a basis, the lawmakers' rationale for patient participation in the G-BA can therefore be summarised as follows (Chap. 1) (HTAi 2014):

- **Relevance** The *relevant patient organisations* and their member organisations are broad based. This allows relevant patient experience and perspectives to become part of HTAs.
- **Fairness** The *relevant patient organisations and their patient representatives* can contribute to the process by having the same right as other stakeholders to initiate a decision-making process (*right to request a decision*) and to take part actively in all consultations and sessions (*consultation right*). Nevertheless, they might not be seen as equal partners by other parties as they do not have comparable organisational, personal and financial support, nor have the right to vote.
- **Equity** Through relevant contributions from the patient perspectives, patient participation helps to reduce underuse, overuse and misuse of health services, to promote quality and more efficient services. This can be considered as contribution to equity within SHI.
- **Legitimacy** Patient participation has contributed to more transparency of G-BA decisions, e.g. by discussing and explaining procedures and decisions within patient organisations. The credibility of G-BA decisions could be further improved, if published justifications of decisions put more emphasis on included patient perspectives (Sect. 25.4.2).

- **Capacity building** The right to initiate HTAs and legally binding decisions by request means that patient organisations can actively contribute to healthcare. Patient participation includes financial, organisational and content support as well as training (Sect. 25.3.3). Empowerment, influence and co-operation of patient organisations and *patient representative*s are promoted.

25.3 Patient Participation in HTA in the G-BA and IQWiG

25.3.1 Patient Participation Throughout the HTA Process

The decision-making process of G-BA can be described with the assessment and appraisal model. Assessments are conducted by a G-BA committee. But generally the G-BA commissions the IQWiG to produce an HTA report. Appraisal of the results takes place in the G-BA subcommittees and working groups where resolution proposals are drafted for the decision-making body which takes a decision in a public session. *Patient representatives* and patient organisations are involved in the entire process in the G-BA.

The IQWiG includes patients or *patient representative*s for the preparation of HTA reports according to its methods papers (IQWiG 2016) (Fig. 25.2).

As an example, we use a request of the *relevant patient organisations* for HTA and benefit coverage of the *Newborn Screening for Critical Congenital Heart Disease Using Pulse Oximetry* from 6 September 2012 (Relevant Patient Organisations 2012) in Table 25.1 and Sect. 25.3.2.

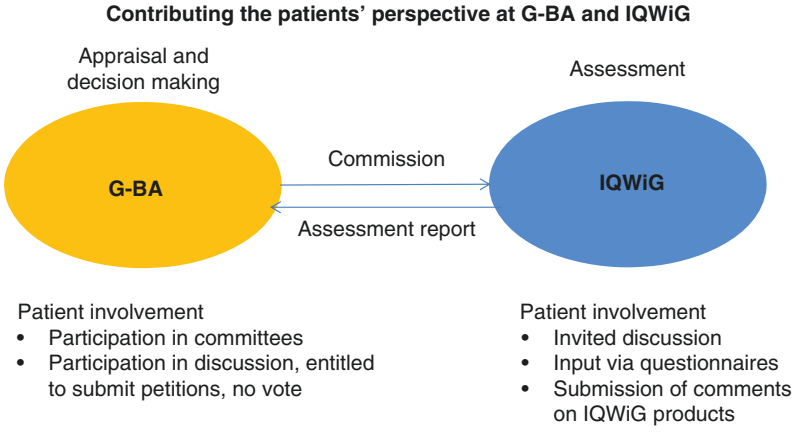

Fig. 25.2 Contributing the patients' perspectives at G-BA and IQWiG (Wieseler 2015)

Table 25.1 Patient participation in G-BA HTA and decision-making process

HTA phase	Relevant patient perspectives	Type of patient participation mechanism
Topic proposal and request by patient organisations		
Indications of deficiencies in care Inclusion into SHI services	Medical care aspects Patient safety Benefit in patient-relevant endpoints (mortality, morbidity, quality of life) For example, – *Congenital heart disease most common organ malformation of newborns* – *Need of fast intensive care of newborns with critical congenital heart disease* – *Improved survival with early diagnostic*	**Any patient group** can *propose topic* to **relevant patient organisations** For example, *Federal Association of Children with Heart Disease* *The G-BA patient involvement specialist team* supports patient groups to develop a draft request meeting G-BA conditions for HTA **Relevant patient organisations** decide to submit the *request* to the G-BA
Topic selection and initiation of HTA (G-BA)		
Acceptance of request, clarification of topic, prioritisation	Collection of detailed information on the topic Healthcare relevance for patient groups (e.g. disease burden, anticipated benefit, available alternatives)	**Patient representatives** *participate* in G-BA committees, with support from G-BA patient involvement team For example, *representatives from the Federal Association of Children with Heart Disease as well as patient representatives from other organisations in the area early diagnosis of children*
Public notice of assessment and appraisal	Initial evaluation of topic (PICO)	Inter alia: **umbrella organisations of self-help groups and patient representatives** *Statement* via questionnaire
Commission to IQWiG for HTA report	Specification of HTA questions For example, *earlier identification and better prognosis of congenital heart disease?*	**Patient representatives:** *participation* in G-BA committees
Early phases of HTA report (IQWiG) (IQWiG 2015b)		
Research question	Patient-relevant endpoints Subgroups, if needed	**Patients, affected persons:** *consultation* via interviews For example, *patient representatives from Federal Association of Children with Heart Disease, parents' initiative of children with heart disease cologne, Herzkind e.V.*

(continued)

Table 25.1 (continued)

HTA phase	Relevant patient perspectives	Type of patient participation mechanism
Preliminary report plan	Patient-relevant endpoints Inclusion and exclusion criteria	**Public, including patient organisations:** *written comments (hearing)*
Preliminary report	Methodical approach and findings	**Public, incl**uding **patient organisations:** *written comments (hearing)*
Appraisal and decision (G-BA)		
Appraisal	Medical relevance, disease burden, access, alternatives	**Patient representatives:** *participation* in G-BA committees **Relevant patient organisations:** may submit (dissenting) *draft decision as request*, if applicable
Decision on inclusion as SHI service in public session		**Patient representatives: participation** Presentation of position or *request* in public session

Adapted from Table 5.3 with example from the assessment and appraisal of the *Newborn Screening for Critical Congenital Heart Disease Using Pulse Oximetry* (G-BA 2016b)

25.3.2 Involvement Mechanisms

Table 25.1 shows patient participation rights and opportunities throughout the HTA process. They involve different actors.

25.3.2.1 Initiation of HTA

The right to request a decision of the *relevant patient organisations* goes beyond a topic proposal that could be considered or not. A request is a formal procedural right. Topics addressed in a request must be discussed, and a formal decision must be made about whether to go ahead with the topic or not.[3] However, once the conditions for an HTA have been met by a request, the G-BA has to initiate the assessment and appraisal procedure, take the topic forward as an HTA and at the end of procedures has to decide about benefit coverage.[4]

Conditions for an HTA are specified in the G-BA rules of procedure. The request has to include a description and substantiated justification with information on the benefits, the target population, medical necessity and cost-effectiveness of

[3] The G-BA is required to discuss requests in the next session of the relevant committee, and, if no decision is possible, define the further proceedings (Social Code Book V. Section 140f 2015d).

[4] Prioritisation is restricted: a decision on acceptance of a request for HTA of non-medicine technology has to be made after 3 months, and process shall be finished after 3 years (Social Code Book V. Section 135 2015b).

the method to be discussed, as well as comparisons to methods already available. Available studies must permit a discussion of the topic, and the request must specify the topic's level of urgency.

Topic proposals are discussed within patient organisations first. Any patient organisation can propose a topic to the *relevant patient organisations*, regardless of its size, funding or skills. There is no paper form for requests, but the G-BA patient involvement specialist team has been established to support with drafting requests. It can review information and existing evidence and estimate the chances of success. On the basis of the collected information, the four *relevant organisations* decide whether to submit a request.

Our example *Newborn Screening for Critical Congenital Heart Disease Using Pulse Oximetry* was proposed by the Federal Association of Children with Heart Disease, a member organisation of the German Disability Council. The Association had support from medical experts, such as the German Association of Paediatric Cardiology. The G-BA patient involvement specialist team reviewed the information and drafted the request according to G-BA conditions, and after internal discussion and decision, *the relevant patient organisations* submitted the request to the G-BA.

25.3.2.2 Patient Participation During the HTA Process

Appointments of patient representatives by the *relevant patient organisations* to G-BA committees are dependent on the experience and competence of the *patient representative* as well as on a committee's agenda.

Each appointment is *topic related*, depending on the individual's knowledge and experience. If a committee works on several issues simultaneously, several persons will be appointed to cover all topics.

In addition to these *topic-related* representatives, the *relevant patient organisations* delegate *permanent patient representative*s with methodological or general expertise. These persons have the additional task to ensure the continuity of the representation of interests, coordinate and support the topic-related representatives and act as a liaison until the G-BA takes a decision.

The number of *patient representatives* per committee ranges from 1 to 12.

In our example the *relevant patient organisations* appointed two topic-related *patient representative*s from the Association of children with child disease into the G-BA. The topic has been discussed in the responsible subcommittee 'Methods Assessment' and its working group 'Children' where *patient representative*s from other patient organisations also permanently work on topics of early diagnosis of children.

Conflicts of interest Before an appointment, the *patient representative*s have to fill out a questionnaire to disclose any potential personal conflicts of interest or those of their organisation. If any conflict of interest is present, the *relevant patient organisations* do not appoint the person to a committee.

Representatives who are appointed must also fill out a disclosure statement with the G-BA of any conflicts of interest regarding the issue under discussion. This disclosure statement can be viewed by all committee members (G-BA 2014).

25.3.2.3 Patient Participation at the IQWiG

Consultation At the start of a commissioned benefit assessment, IQWiG grants additional participation opportunities that are not guaranteed by law. This is to allow patient perspectives to be taken into consideration for the formulation of the research question and further assessment. This participation takes the form of a consultation or a questionnaire with persons affected who can be patients or their relatives with individual experience, as well as *patient representative*s with collective experience. The recruitment takes place via the *relevant patient organisations* and their member organisations, or the IQWiG can contact those persons directly (IQWiG 2015b) (IQWiG 2015a).

In the course of our example *patient representative*s from the Federal Association of Children with Heart Disease, the parents' initiative of children with heart disease cologne and Herzkind have been consulted regarding the quantity of benefit (quality of life), alternatives, possible negative consequences for parents and children, treatment options as well as efficiency.

Conflicts of interest Each person involved at the IQWiG must also fill out a form to disclose any potential conflict of interest. The IQWiG publishes whether a question was answered with 'yes' or 'no' (IQWiG 2015a, 2015b).

Statements The law ensures the right of *relevant patient organisations* to submit written statements at various stages of an HTA assessment in the IQWiG (Social Code Book V. Section 139a 2015c), e.g. after the publication of preliminary report plans and preliminary reports. However, the IQWiG opens the hearing procedures to the whole public including private persons.

Research The IQWiG has conducted research with two pilot studies in order to provide evidence about patients' preferences (Chap. 11). These approaches have not yet become regular instruments of patient involvement in HTA under the responsibility of the G-BA.

25.3.3 Measures to Support Patient Participation

Patient participation in the G-BA and IQWiG is mainly based on volunteer work by *patient representatives*. Therefore, supportive measures are required to be able to exercise rights effectively.

Statutory measures:

- Reimbursement of travel expenses, compensation for loss of earning up to a maximum sum and lump sum as representation allowance (Social Code Book V. Section 140f 2015d).
- The G-BA patient involvement specialist team is tasked with organisational and content support (Social Code Book V. Section 140f 2015d): this includes methodological and legal advice, help with consulting documents, organisation of meetings and support with the nomination procedures for *patient representatives*. Training is also provided by the G-BA Medical Consultancy Department.

- Embedding patient participation and procedural rights in the G-BA by-laws (Social Code Book V. Section 91 2015a).
- Barrier-free access to offices and rooms, consultation documents, website and organisation of meeting assistance (Gesetz zur Gleichstellung behinderter Menschen 2002) (G-BA 2008).

Additional measures:

- Transparent internal appointment and collaboration rules for the *relevant patient organisations* and *patient representatives*.[5]
- Participation in events, also as speakers, facilitators or participants in discussions.
- In 2016 the G-BA patient involvement team established an online portal for communication, joint work and education (G-BA 2016a).

25.4 Evaluation and Challenges

25.4.1 Issues in Evaluating Patient Participation

So far, no comprehensive scientific evaluation of patient involvement in the G-BA and IQWiG has been conducted. Challenges for evaluation are the various forms and actors of participation, as well as the political aspects influencing G-BA decisions.

Requests for an HTA are published on the G-BA website as soon as a decision-making process is initiated, so that after some time, even years, the impact could be examined by comparing the intent of the request with the G-BA decision and further implementation into healthcare.[6]

The circumstances surrounding how *consultation right*s are exercised by *patient representative*s in the G-BA committees are more difficult. The workflow is a joint consultation in the preparatory committees aimed at achieving a consensual reconciliation of interests among the various healthcare stakeholders. Individual statements expressed or submitted by the *patient representative*s in the meetings become part of the discussion and can influence the votes of the other persons involved and thus also decisions. But this influence can hardly be evaluated. The contributions of the *patient representative*s might be recorded in the minutes, but the consultations and the relevant documents of the non-public meetings are confidential.

[5] By-laws of the patient representative coordination committee of 21 November 2012, not published

[6] Requests and documented contributions by *relevant patient organisations* and *patient representatives* can be found on the G-BA website (www.g-ba.de). The website of the G-BA patient involvement specialist team (https://patientenvertretung.g-ba.de) will also include all requests formally submitted by the patient representatives, along with the consultation proceedings.

No minutes are taken of consultations on patient-relevant endpoints that the IQWiG conducts with persons affected (IQWiG 2015b).

What remains are pertinent quotes by the impartial chairs of the G-BA. On the occasion of his retirement as the former chair of the G-BA in 2012, Rainer Hess expressed: 'The G-BA is unviable without the patient representatives' (Gerst 2012). And the current impartial chair, Josef Hecken, confirmed in an interview: 'I consider the involvement of the patient representatives in its current form to be exceptionally helpful because it brings the specific perspectives of the patient into the discussion in a highly responsible manner' (Gottfried and Kessen 2012).

25.4.2 Future Challenges

Patient participation in the G-BA including *relevant patient organisations* and appointed *patient representatives* with different perspectives makes it possible, not only to reflect the overall acceptance of HTA and decision-making from a citizen's point of view but also to include patient experience and to organise patient advocacy.

The establishment of the patient involvement specialist team in the G-BA in 2008 has become a powerful support. However, significant tasks fall to the patient organisations. These include, in particular, recruiting volunteer *topic-related patient representatives* for the indications addressed in an HTA and appointing *permanent patient representatives*.

So far, the state has shied away from providing large subsidies for patient organisations out of a concern of intervening in the traditional balance of interests between health insurance funds and care providers. However, a statement in a ruling by the Federal Constitutional Court on 10 November 2015 has attracted much attention. The court stated that it cannot be ruled out that the constitutional legitimacy of the G-BA as self-governing committee might be missing, if it regulates with high intensity the affairs of others who are not permitted to contribute to the process (Bundesverfassungsgericht 2015). This has been commented on in the jurisprudential literature: 'Better consideration of the interests and rights of insured persons, patients, and third parties affected belongs at the top of the political agenda, […]' (Gassner 2016).

Over the past years, lawmakers have assigned more and more decision-making power to the G-BA. Its decisions have a huge impact on healthcare, and they are being closely observed by the Federal Ministry of Health, politics, courts and the public.

Decisions of the G-BA have a good level of transparency. They are published on G-BA's homepage including rationales and documentations of the HTA process. But more and more, the acceptance of decisions will also depend on the ability and commitment of the G-BA to describe how patient perspectives have been determined, involved and considered.

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References

Bundesarbeitsgemeinschaft der PatientInnenstellen. Website national association of patient advise centres and action groups. 2016. http://www.bagp.de/. Accessed 03 Aug 2016.

Bundesverfassungsgericht. Federal Constitutional Court, Ruling of 10 Nov 2015, file ref. 1 BvR 2056/12, marginal reference 22. 2015. http://www.bundesverfassungsgericht.de/SharedDocs/Entscheidungen/DE/2015/11/rs20151110_1bvr205612.html. Accessed 28 Jul 2016.

Deutsche Arbeitsgemeinschaft Selbsthilfegruppen. Website German association of self-help groups. 2016. http://www.dag-shg.de/. Accessed 03 Aug 2016.

Deutscher Behindertenrat. Action alliance of German organisations of persons with disabilities and (chronic) diseases. 2016. http://www.deutscher-behindertenrat.de/. Accessed 03 Aug 2016.

Draft SHI Modernisation Act. Entwurf eines Gesetzes zur Modernisierung der gesetzlichen Krankenversicherung, Bundesdrucksache 15/1525. 2003a. p. 106f.

Draft SHI Modernisation Act. Entwurf eines Gesetzes zur Modernisierung der gesetzlichen Krankenversicherung, Bundesdrucksache 15/1525. 2003b. p. 72f.

Draft SHI Modernisation Act. Entwurf eines Gesetzes zur Modernisierung der gesetzlichen Krankenversicherung; Bundesdrucksache 15/1525. 2003c. p. 128f.

Gassner U. Götterdämmerung des Gemeinsamen Bundesausschusses. NZS. 2016;4:122–4.

G-BA. Federal Joint Committee statement of intent to promote barrier-free–Absichtserklärung des Gemeinsamen Bundesausschusses zur Förderung von Barrierefreiheit. 2008. https://www.g-ba.de/informationen/beschluesse/665/. Accessed 28 Jul 2016.

G-BA. The legal status of the Federal Joint Committee. 2013. http://www.english.g-ba.de/downloads/17-98-2850/GBA_Rechtsstellung_08-04-2013_engl.pdf. Accessed 03 Aug 2016.

G-BA. Rules of procedure. 2014. https://www.g-ba.de/downloads/62-492-1002/VerfO_2014-12-18_iK-2015-04-16.pdf. Accessed 03 Aug 2016.

G-BA. Patient participation in the G-BA. 2016a. https://patientenvertretung.g-ba.de. Accessed 28 Jul 2016.

G-BA. Subjects of consultations in the area of child and youth prevention—Beratungsthemen zu "Kinder- und Jugendprävention". 2016b. https://www.g-ba.de/informationen/beratungsthemen/zum-aufgabenbereich/28/#details/1768. Accessed 28 July 2016.

Gerst T. Gemeinsamer Bundesausschuss: viel Arbeit zum Abschied vom Amt. Dtsch Arztebl. 2012;109(26):A-1349.

Gesetz zur Gleichstellung behinderter Menschen. German equal opportunities for people with disabilities act. 2002.

Gottfried M, Kessen A. Interview with Josef Hecken–Selbstverwaltung ist besser als jedes andere Modell. Ersatzkasse magazin. 2012;9–10:11.

HTAi. Values and quality standards for patient involvement in HTA. 2014. http://www.htai.org/fileadmin/HTAi_Files/ISG/PatientInvolvement/v2_files/Info/PCISG-Info-ValuesandStandards-30-Jun14.pdf. Accessed 03 Aug 2016.

HTAi. Patient and citizen involvement. FAQ for HTA agencies and Policy Makers; Question 5: Who can provide the patient perspective in HTA processes? 2015. http://www.htai.org/interest-groups/patient-and-citizen-involvement/resources/for-hta-agencies-and-policy-makers/faq-for-hta-agencies-and-policy-makers.html. Accessed 03 Aug 2016.

IQWiG. Involvement of people affected in the dossier assessment. 2015a. https://www.iqwig.de/download/Involvement_of_people_affected_dossier_assessment.pdf. Accessed 28 July 2016.
IQWiG. Involvement of people affected in the production of reports on benefit assessments, Vers. 1.0. 2015b. https://www.iqwig.de/download/Involvement-of-people-affected_benefit-assessment.pdf. Accessed 28 July 2016.
IQWiG. General methods, Vers.4.2. 2016. https://www.iqwig.de/download/IQWiG_General_Methods_Version_%204-2.pdf. Accessed 28 July 2016.
National Association of Statutory Health Insurance Funds. Website GKV-Spitzenverband. 2016. https://www.gkv-spitzenverband.de/english/statutory_health_insurance/statutory_health_insurance.jsp. Accessed 28 July 2016.
Patient Involvement Act. Verordnung zur Beteiligung von Patientinnen und Patienten in der gesetzlichen Krankenversicherung–Patientenbeteiligungsverordnung; Bundesdrucksache 899/03. 2003.
Relevant Patient Organisations. Antrag der Patientenvertretung nach § 140f SGB V, Screening auf schwere congenitale Herzfehler mittels Pulsoxymetrie nach § 26 SGB V (Request for decision on newborn screening for critical congenital heart disease using pulse oximetry). 2012. https://www.g-ba.de/downloads/40-268-2400/2012-11-22_Einleitung-Beratungsverfahren-Pulsoxymetrie_Antrag-PatV.pdf. Accessed 05 Aug 2016.
SHI Modernisation Act. Gesetz zur modernisierung der gesetzlichen krankenversicherung. In: Bundesgesetzblatt, BGBL I Nr. 55. Bonn: Bundesanzeiger; 2003.
Sozialgesetzbuch Fünftes Buch. Social Code Book V. Section 12 efficiency demand. 1992.
Sozialgesetzbuch Fünftes Buch. Social Code Book V. Section 135. Assessment of diagnostic and treatment methods. 2015b.
Sozialgesetzbuch Fünftes Buch. Social Code Book V. Section 139a. Institute for Quality and Efficiency in Health Care. 2015c.
Sozialgesetzbuch Fünftes Buch. Social Code Book V. Section 140f. Participation of interest representation of patients–Beteiligung von Interessenvertretungen der Patientinnen und Patienten. 2015d.
Sozialgesetzbuch Fünfte Buch. Social Code Book V. Section 70. Quality, humanity and economic effeciency. 1999.
Sozialgesetzbuch Fünftes Buch, Social Code Book V. Section 91. Federal Joint Committee. 2015a.
Verbraucherzentrale Bundesverband. Website federation of German consumer organisations. 2016. http://en.vzbv.de/. Accessed 03 Aug 2016.
Wieseler B. Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen. Engagement with patients–HTA processes. EMA/EUnetHTA Meeting; 23 Nov 2015; Copenhagen. 2015.

Chapter 26
Italy

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26.1 Introduction

In the field of medical devices, HTA activities in Italy are conducted by the National Agency for Regional Health Services (AGENAS), while pharmaceuticals are assessed by the Italian Medicine Agency (AIFA). Italy has a regionally organised national health service, and regions are responsible for organising and delivering healthcare, while, at the national level, the Ministry of Health (MoH)—supported by specialised agencies—sets the fundamental principles and decides the core benefit package, allocating national funds to regions (Ferré et al. 2014). This chapter focuses on AGENAS activities undertaken over the past 10 years to involve patients in HTA and promote this approach across regions, including primary and secondary research and education and training of patient organisations to build capacity. It highlights the critical role played by international HTA bodies, a supportive national policy context in driving patient involvement in HTA and the importance of activities that address concerns about patient involvement among HTA professionals.

26.2 Policy Context, Barriers and Drivers to Involving Patients

The National Health Plan[1] 2006–2008 explicitly highlighted for the first time that HTA was an important tool for healthcare decisions. Up to that point, very few regions had used HTA in their healthcare decision-making processes, and those that

[1] This is the main instrument for healthcare planning at a national level. It is drafted by the MoH following consultations with the regions, and it is approved by the government in agreement with the State-Regions Conference.

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had used a variety of approaches. As a result, in 2007, the Standing Conference between state, regions and autonomous provinces[2] gave AGENAS the remit of supporting regions to develop HTA activities.

AGENAS started developing a series of initiatives such as networking with scientific societies, both on an international and national level (HTAi and the Italian Society for HTA (SiHTA)), participating in EUnetHTA and producing HTA reports on request by the general directorate of medical devices. In 2010–2011, AGENAS formed the Italian Network for HTA with Italian regions (RiHTA) to provide HTA methodologies, create networking and training events and implement a model of collaboration with regions based on the joint production of HTA reports.

During this period (2007–2013), AGENAS, while establishing its role in HTA and with regions, also attempted to improve patient involvement in HTA. It assessed patients' views by using primary and secondary qualitative research and supported an educational programme in HTA for citizen and patient organisations to overcome barriers and identify drivers to help them take part in HTA.

With the latest Pact for Health 2014–2016 (agreement between the national government and regional governments), that informed the Law 190/2014, Italy established HTA for medical devices at the national level. According to the law, AGENAS is responsible for coordinating the new HTA national programme for medical devices. A committee—the Cabina di Regia (CR) composed of representatives from the MoH, regions, AGENAS and AIFA—has been established to set the strategic priorities of HTA activities at national, regional and local level. This later policy development seemed to give more attention to the principles of stakeholder involvement in HTA, as new regulations also provided for the establishment of the Innovation Working Group (IWG) (Tavolo sull'Innovazione) with an advisory role for the CR composed of HTA stakeholders: representatives of citizens' associations, clinicians, universities and industries. This intention was partly reflected in AGENAS HTA activities, which could undertake additional work to support patient involvement in HTA and resulted in the publication of the handbook of AGENAS HTA procedures and piloting of the HTAi *Patient Group Submission Template*.

26.3 From Analysis of Patients' Views to Participation of Patient Associations

26.3.1 Evaluating Patient Issues with Primary and Secondary Qualitative Research

In its earliest HTA reports, the AGENAS rationale for including patients' views was mainly scientific. Some researchers stressed the importance of this type of analysis since it provided an irreplaceable view and brought diverse evidence to the

[2] This body was established as a permanent interface for consultation and communication between the state and the regions in the domains of public policy where their mandates overlap.

assessment. At the outset, this evidence was collected by reviewing existing literature on patients' views or undertaking primary research. However, the introduction of this kind of evidence in HTA reports was not accepted by all those involved in HTA.

A key barrier was the widespread lack of confidence in patient-based evidence and qualitative research in a field where the predominant epistemological paradigm is the one from biomedical sciences. A key driver to overcome this barrier was the link AGENAS built with the HTAi Interest Group and EUnetHTA, since the HTA Core Model® dedicated an entire domain to social impact and patients' views (see Chap. 2410.1007/978-981-10-4068-9_6). The fact that those influential organisations gave attention to patients' experiences in HTA and set a methodological point of reference for patient aspects analysis provided more credibility in conducting this type of research.

In individual HTAs, when relevant, AGENAS has dedicated a chapter to patients' aspects that includes a literature review of studies on patient acceptability/views or primary data collected using traditional non-participatory social research techniques. This was the case of two HTA reports, the first one being produced entirely by AGENAS (2008), while the second one was produced together with several regions (Lo Scalzo et al. 2012).

For the first report on *wireless capsule endoscopy* (WCE), no studies were retrieved on patients' views; therefore, we collected primary data on preferences for WCE compared with other technologies via a questionnaire. This was administered to a sample of 127 patients from five Italian centres providing WCE.[3] The second report was about *new devices for young diabetics* (Lo Scalzo et al. 2012). In this case, we found a good amount of qualitative and quantitative literature on patients' views. We reviewed this literature and the results were reported in a specific chapter on patients' aspects within the report.

At this time, there was no patient participation in the HTA topic proposal phase, nor was any direct input allowed in any other phase with the exception of the final one, when the final draft HTA report underwent public consultation on the MoH's website. However, that kind of involvement relied too much on the patient organisations' being proactive, which cannot be taken for granted as they may not be aware of the consultation.

26.3.2 Building Capacity: HTA Education Programmes

Initially, a barrier to thorough patient participation in the HTA process was an absence of any previous experience of the AGENAS HTA unit in interacting with patients' and citizens' associations. In addition, a certain lack of confidence might have been due to a perception that patient and citizen organisations are potentially influenced by hidden interests and hard to deal with due to their challenging

[3] See Chap. 6.2 in an assessment of patients' acceptability of the WCE procedure in AGENAS 2008.

advocacy standpoint. HTA is also very technical and patients can be perceived as too emotional when making their point. Additionally, there were concerns about how much time and resources would be spent managing patient organisations' input. This initially discouraged involvement activities.

To overcome these barriers, AGENAS built on the growing culture of participation among Italian citizens' and patients' associations. These associations proposed that the main public institutions involved in HTA should organise an HTA educational programme for lay people. A turning point for patient organisation involvement in HTA was reached in 2013 when the first summer school for civic leaders in HTA was proposed by relevant citizens' organisation, Cittadinanzattiva (Active Citizenship). The educational programme they proposed was sponsored by AGENAS, SiHTA and the Italian Federation of Healthcare and Hospital Trusts (FIASO), which since then have been active within the scientific coordination committee of the school. The summer school is now in its fourth edition.

Run by senior HTA staff and international experts, the summer school format provides two residential training modules for a total of 6 days of lessons. Each module is separated by a 1-month break during which participants work on different homework projects. For example, in the first editions, they were asked to compile patient input using the *Checklist for the Content of Patient Evidence* from the guide *Understanding Health Technology Assessment* (Health Equity Europe 2008). Initially, participants came from national and/or local patient associations and Cittadinanzattiva's regional and national sections. In the subsequent summer schools, the students included public institution participants who were active in HTA at the local level with the objective of facilitating mutual knowledge among patient/citizen organisations and public institutions.

In the 2016 edition, the school promoted patient involvement approaches across regions. Indeed, a recent national survey has shown that stakeholder involvement is declared to be performed by five regions and just three regions out of 21 state that they also involve patient associations (Cerbo et al. in press). For this reason, the school focused on participants from three northern regions and autonomous provinces, and classes were organised and hosted in this area, rather than Rome.

The school is important as it allows citizen and patient groups and the main institutional HTA stakeholders to meet each other and overcome many of their concerns about each other. Moreover, participants learn HTA objectives, language, methodology and how to provide patient input to HTA. Cittadinanzattiva, together with school's participants, wrote, for example, guidelines on how to be involved in HTA (Terzi 2014) and recommendations on the best criteria to choose associations to be involved in HTAs (Cittadinanzattiva 2015). From the AGENAS perspective, this was a unique opportunity to build knowledge of patient associations, with Cittadinanzattiva acting as an umbrella association, gatekeeper and interlocutor.

26.3.3 The AGENAS HTA Procedures Handbook and the HTAi Template

A changing policy context in 2014, new legislation requiring AGENAS to coordinate the HTA national programme for medical devices and encouragement of stakeholder involvement (see IWG) have probably acted as a further driver and given more legitimacy to the principles and procedures of patient involvement in HTA. In fact, in regard to the overall HTA process, since 2015, any member of the public has been able to propose technologies for assessment via the AGENAS website, while in the beginning (2008), this was only possible for the emerging technologies and horizon scanning programme.

In 2014 AGENAS published its HTA procedures handbook (AGENAS 2014). It describes the important phases of including patients' views from published research and systematically obtaining input from patient associations, and a procedure is proposed to involve patient associations in HTA when relevant.

Nonetheless, the handbook's procedure is provisional and needs to be revised in light of the results of the pilot AGENAS which was developing at the time of its publication. The pilot aimed to test a new tool for patient association involvement, the *HTAi Patient Group Submission Template for HTA*, in a report on dialysis modalities (Gillespie et al. 2015). We provided the template to the patient associations appointed by the umbrella organisation, Cittadinanzattiva and its Tribunale per I Diritti del Malato (TDM), so they could collect input. AGENAS relied on a pre-existing collaboration agreement with Cittadinanzattiva (stipulated after a public call) for the implementation of the Pact for Health 2014–2016. Cittadinanzattiva and TDM staff were ideal interlocutors, since as promoters of the summer school for civic leaders in HTA, they were well trained in HTA.

The HTAi template was translated by AGENAS into Italian and adapted to the HTA report's specific information needs: to understand patients' experiences with different dialysis modalities and any delivery problems at regional level. Some questions were thus reformulated, others were cut and two different versions of the template were used. One was tailored to patient organisations' representatives and the other to individual patients selected with a purposive sampling procedure which relied on the active participation of the patients' representatives involved.

TDM was asked to find representatives from patient organisations who could work with AGENAS to identify other relevant dialysed patient organisations to be involved. AGENAS worked with the vice president of the national forum of the nephropathic–transplanted people and identified a list of four associations from the forum on the basis of their geographical location and the typology of patients represented (e.g. representative of each dialysis modalities under assessment).

Each patient organisation's representative had to complete the first template and administer the second one to, at least, one patient for each five dialysis modalities. AGENAS staff supported them so that they, in turn, could provide support to patients

completing the templates. They collected and returned all completed templates to AGENAS. Researchers performed a thematic analysis of all the answers received, and the synthesised input was introduced in the report as a paragraph within the chapter on Patients Aspects. The patients' experiences almost corresponded to the ones found in the systematic review of the qualitative literatures. However, the templates from patient organisations' representatives revealed an important problem of equity in access to different dialysis modalities across regions, which was highlighted in the final HTA report recommendations.

One of the template's limitations was related to the auto-administration. In some cases, it is probable that a lack of familiarity with communicating one's views and experiences in writing affected the informative power of the survey. Moreover, this pilot demonstrated the need for a more inclusive involvement procedure, as it was revealed that some important national patient associations were excluded from the initial work. They were later invited to review the document after making complaints.

26.4 Conclusion

Over the past decade, AGENAS undertook a series of activities to progress patient involvement in HTA and identified barriers, challenges and drivers. Ultimately, the success of its patient involvement activities depended on the policy context, which gave it a mandate to support patient participation.

AGENAS' first step was to introduce patients' views analysis in HTA reports via primary and secondary qualitative research. The issue of epistemological distrust towards this kind of research was in part addressed by the remit AGENAS gained from the State and Regions Conference about HTA and the resulting commitment to EUnetHTA and HTAi which established a methodological foundation.

Perceptions of patient associations were a barrier to participation in the HTA process. The proactive role of patient and citizen associations in proposing and organising the summer school for civic leaders helped to overcome this. Moreover, AGENAS also adopted procedures to manage any involved stakeholders' conflict of interests.

The recently published AGENAS handbook on HTA procedures provides principles, methods and procedures for patients' views analysis and patient association involvement. It can be seen as the organisation's commitment towards patients' active participation in HTA. This was in part facilitated by the latest policy context developments which provide a future major role for AGENAS in HTA and the new HTA national programme.

Future developments will be related to the use of the template to engage patient associations and the promotion of patient involvement models at a regional level, including via the summer school for civic leaders. Nonetheless, the success of any future public involvement in HTA relies on a national policy context that facilitates this by explicitly stating the need of patient involvement. A useful first step is the

presence of a citizen association in the IWG. A similar explicit commitment at European level (e.g. by EUnetHTA and the HTA Core Model) is important since international organisations have an authoritative role in setting the best principles and practices of HTA and can drive national best practices.

As transparency and inclusiveness increasingly become leading principles of any public policy at European and national level, and patient organisations grow and push for opportunities to participate in processes, health organisations at national and regional level will continue the journey towards patient involvement in HTA.

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References

AGENAS, Agenzia Nazionale per i Servizi Sanitari Regionali. Wireless capsule endoscopy in the diagnosis of small bowel disease. 2008. http://www.salute.gov.it/portale/temi/documenti/dispositiviMedici/C_17_pagineAree_1202_listaFile_itemName_3_file.pdf. Accessed 20 Oct 2016.

AGENAS, Agenzia Nazionale per i Servizi Sanitari Regionali. Manuale delle procedure HTA (HTA procedures handbook). 2014. http://www.agenas.it/images/agenas/hta/Manuale_procedure_HTA.pdf. Accessed 25 Oct 2016.

Cerbo M, Cicchetti A, et al. Indagine conoscitiva sulle attività di HTA in Italia. Rome: AGENAS; in press.

Cittadninzattiva. La Rilevanza delle organizzazioni civiche nei processi di HTA. Verso la Raccomandazione Civica. 2015.http://www.cittadinanzattiva.it/files/progetti/salute/hta/Raccomandazione_civica.pdf. Accessed 02 Nov 2016.

Ferré F, de Belvis AG, Valerio L, Longhi S, Lazzari A, Fattore G, Ricciardi W, Maresso A. Italy: health system review. Health Syst Transit. 2014;16(4):1–168.

Gillespie F, Amicosante AMV, Lo Scalzo A, Chiarolla E, Ondei P, Paone S, Remuzzi G, Santoro A, Teatini U, Jefferson T, Cerbo M. Valutazione HTA delle Modalità di dialisi in Italia—AGENAS. 2015. http://www.salute.gov.it/imgs/C_17_pagineAree_1202_listaFile_itemName_9_file.pdf. Accessed 10 Aug 2016.

Health Equality Europe. Understanding Health Technology Assessment (HTA). 2008. http://www.htai.org/fileadmin/HTAi_Files/ISG/PatientInvolvement/v2_files/Resource/PCISG-Resource-HEE_ENGLISH_PatientGuidetoHTA_Jun14.pdf. Accessed 25 Oct 2016.

Lo Scalzo A, Lenzi L, Chiarolla E, Maltoni S, Negro A, Ballini L, et al. HTA report: new devices for the management of glycaemia in young diabetics. 2012. http://www.salute.gov.it/imgs/C_17_pagineAree_1202_listaFile_itemName_23_file.pdf. Accessed 11 Aug 2016.

Terzi A. Health Technology Assessment e organizzazioni civiche. Linee guida per l'intervento. 2014. http://www.cittadinanzattiva.it/files/progetti/salute/hta/Linee_guida_HTA_per_organizzazioni_civiche.pdf. Accessed 2 Nov 2016.

Chapter 27
Scotland

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27.1 Introduction

Patient involvement has been a feature of Health Technology Assessment (HTA) in Scotland since a national body to assess the clinical and cost-effectiveness of health technologies was established in 2000. The impetus for involving patients in HTA may be linked to the Scottish Parliament's principle of encouraging public participation in decision-making (The Scottish Parliament 1999) and a UK-wide push for greater transparency and patient involvement in healthcare following an inquiry into a serious failure in health services (Bristol Royal Infirmary Inquiry 2001). As a result, Scotland followed the wider Danish model for HTA (Chap. 22) that evaluated organisational and patient issues, alongside clinical and cost-effectiveness. HTA is now undertaken for the National Health Service in Scotland (NHSScotland) by two bodies within Healthcare Improvement Scotland (HIS).[1] The Scottish Medicines Consortium (SMC) appraises medicines, and the Scottish Health Technologies Group (SHTG) appraises all other health technologies (non-medicine technologies). This chapter provides an overview of the development of patient involvement in HTA in Scotland, contrasting approaches for rapid and full HTA of medicine and non-medicine technologies. It highlights the potential value of patient group submissions, patient involvement advisory groups and Public Partners and

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includes a case study from a full HTA demonstrating the impact of qualitative research on patient issues. The chapter concludes with reflections on outstanding challenges and future plans.

27.2 Policy Context

A culture of involvement in NHSScotland has been strengthened over the past 15 years with a range of policy initiatives. In 2001, a national strategy called for individuals, groups and communities to be involved in improving the quality of healthcare, influencing priorities and planning services (Scottish Executive 2001). In 2005, the Scottish Health Council was established to promote patient focus and public involvement in the NHS. This was followed by a quality strategy (The Scottish Government 2010) and *Patient Rights Act* (The Scottish Parliament 2011) that place a responsibility on the NHS to encourage, monitor, take action and share learning from the views they receive and gives people a legal right to give feedback, make comments, raise concerns or make complaints about NHS services.

27.3 Full HTAs: All Health Technologies

Initially, HTA in Scotland was undertaken by the Health Technology Board for Scotland (HTBS). HTBS developed processes that encouraged patient participation throughout HTA to ensure that 'issues that matter to patients may be understood and used to inform assessments' (HTBS 2002). Its approach to patient involvement was of continuous learning and included:

- Finding ways to enable patient groups to take part according to their ability (e.g. see approaches in Slattery et al. 2003)
- Establishing a patient involvement advisory group with representatives from umbrella patient groups, academics and local health groups
- Informing relevant patient groups about participation in HTA
- Inviting submissions about patient experiences of the technology or its comparator, views on its advantages/disadvantages and issues particular to Scotland such as access, travel and variations in cost (HTBS 2002)
- Including a representative from at least one relevant patient group in the expert group guiding the HTA from scoping the research questions to appraising and communicating the evidence
- Commissioning literature reviews and primary research to identify patient needs, preferences and/or experiences (e.g. Bradbury et al. 2002)
- Developing a plain language guide for each HTA with relevant patient groups.

This involvement resulted in changes to HTA scopes and research questions. For example, when assessing the organisation of diabetic retinopathy screening, patient involvement highlighted barriers to attendance and refocused the main HTA

question to a key patient issue, the need for potentially inconvenient and uncomfortable eye drops in the screening (Facey et al. 2002).

27.4 Patient Participation in Rapid HTAs of Medicine

27.4.1 Establishment

In 2001, SMC was established within HTBS to conduct HTAs of medicines within 12 weeks. The rapid timeframe was insufficient to include primary or secondary patient issues research, so SMC sought to develop a process that would adequately resource 'active, well-informed patient involvement' (SMC 2002).

Initially, two Public Partners[2] were appointed to SMC, one from an umbrella patient group and the other from a health board patient advisory panel. In August 2002, they joined HTBS staff, the SMC deputy chair and a pharmaceutical company representative to form the Patient and Public Involvement Group (PAPIG). Its aim was to develop processes for SMC which provided an opportunity for every patient group to present their views (SMC 2002). Similar to the way in which manufacturers could submit evidence, a structured template was developed to help patient groups present information about patients' experiences of living with the condition or using the comparator or new medicine. These submissions were given to SMC members and summarised by Public Partners at the SMC meeting. To attract submissions PAPIG undertook promotional activities and established an email alert system to advise relevant patient groups about forthcoming assessments.

Working with the pharmaceutical industry, PAPIG developed a template that a submitting company could complete to provide submitting patient groups with information about the medicine in plain language. Although the Association of the British Pharmaceutical Industry (ABPI) agreed that this would not breach the industry code of practice regarding promoting medicines to the public, some companies remained concerned about their use. As a result, the template was not completed by many companies.

27.4.2 Early Development

After 2 years of operation, SMC had received only 11 patient group submissions, which were of varying quality. Although exemplar submissions were published, it was not until 2013 that SMC included a summary of patient group submissions in its published guidance. The submissions remain confidential, but patient groups are encouraged to publish them on their own websites.

[2] In February 2004 a third Public Partner was added to ensure that at least one Public Partner, and usually two, attended each SMC meeting given that Public Partners often have other responsibilities. For information about Public Partners, see Sect. 6.3.3.

The patient group submission form evolved as feedback was received from patient groups and as Public Partners identified the information that had most influence on the SMC's deliberations. Due to ongoing concerns about potential bias, a declaration of interest section, including details of income from pharmaceutical companies, was added. Increased awareness saw patient group submissions steadily increase to 35 in 2006. However, many patient groups remained unaware of SMC and its processes, and there was no support for those who wanted to make submissions (SMC 2008). So, SMC employed an individual from an umbrella patient organisation to help patient groups complete submissions and offer feedback.

27.4.3 Recent Developments

In 2013, three patient groups submitted petitions to the Scottish Parliament stating that SMC prevented patient access to medicines for rare conditions. National multi-stakeholder reviews were instigated that led to changes in SMC processes. These included holding SMC meetings in public from May 2014 and employing new staff to provide strategic leadership for SMC patient participation activities.

A further review in 2014, with feedback from 54 patient groups, highlighted that patient groups often found their relationship with SMC to be one-sided. SMC relied on them for information, but they found it difficult to get information, support or feedback from SMC. In response SMC agreed an action plan including the development of a Public Involvement Network (PIN) and formalised relationships with submitting patient groups through a registration process to become SMC Patient Group Partners (SMC 2016a). SMC undertook to provide regular training days and publish materials on its website to explain how to create balanced submissions. It contacted relevant patient groups to encourage them to make submission and offered them one-to-one support. To reduce duplication of effort, SMC developed a system to capture core information about each patient group when they registered as a Patient Group Partner. The patient group submission form was substantially simplified, and a simple method guide was created by adapting the HTAi guidance for patient groups on completing a submission template (Chap. 6).

In 2014, SMC saw a 32% increase in patient group submissions and a further 35% increase, to 96 submissions, in 2015.

Since August 2014, pharmaceutical companies have been able to request a Patient and Clinician Engagement (PACE) meeting when orphan or end-of-life medicines are given a New Drugs Committee (assessment) decision of not recommended for use. The PACE meeting seeks to elicit information about the value of the medicine that may not be apparent in the clinical and economic evidence by encouraging dialogue among patient/carer[3] representatives and clinicians with HTA staff. A consensus statement is developed using a standard template (SMC 2016b), a summary of which is read at the outset of the SMC (appraisal) deliberations.

[3] Called caregivers in other chapters in this book

A PIN advisory group was established in 2015, including Public Partners, four Patient Group Partners, an SMC committee member and a local health board representative. In its first year, the advisory group gained a reputation for making balanced and implementable recommendations for strengthening patient/carer input and relationships. As a result of the group's work, SMC decisions are shared in confidence with relevant patient groups 5 days in advance of public release to allow them to prepare media responses and plan services such as briefing helpline staff. Educational videos have been made, presentations of patient group submissions for PACE medicines have been adapted and a mentoring process for PACE has been established. Additionally, a new form to enable the pharmaceutical industry to provide a short overview of key facts about their product to share with patient groups has been codeveloped by patient groups, industry and SMC staff (SMC 2016c).

27.5 Patient Involvement in HTAs for Non-medicine Technologies

27.5.1 Processes

Non-medicine technologies pose challenges for patient involvement. As they can be used for therapeutic or nontherapeutic purposes, patients may not know what technology has been used (e.g. in a surgical procedure). They can cover a wide range of interventions and delivery mechanisms (e.g. medical equipment used in hospitals, diagnostic tests, surgical implants, psychological therapies, educational programmes). In addition, a single technology can be used for a wide range of medical conditions (e.g. magnetic resonance imaging) meaning that there may not be an obvious patient group to engage with. Despite these issues, it would seem essential to assess patients' views on non-medicine technologies, particularly when the patient is the user of the technology (e.g. self-monitoring systems, home devices).

SHTG was established in 2007 to assess non-medicine health technologies. For a few topics, full HTAs are produced, and the timescales and resources available for these offer the opportunity for an in-depth consideration of patients' needs, preferences and experiences. However, in most cases rapid HTAs are undertaken with a systematic review of secondary research on clinical and cost-effectiveness, so achieving effective patient involvement in this process is more challenging.

Patients and patient groups can suggest HTA topics, but to date no proposals have been received. This may be due to the complexity of creating a topic proposal even with the help offered. In the future, SHTG plans to make periodic direct topic calls to targeted patient organisations.

For rapid HTAs, relevant patient groups are asked to provide comments via the peer review process. This approach is not ideal as the questions are not specific to patient groups. However, review of confidential peer review comments has shown that patient groups have contributed important comments about the organisation of services–explaining how Scottish services differ from those described in the

literature and highlighting issues of equity and access. They have also pointed to patient experience surveys and health service audits. For example, patient group comments revealed a major difference in the provision of prostate surgery (open vs. laparoscopic) across Scotland that was highlighted in the Evidence Note (HTA report) conclusions (Healthcare Improvement Scotland 2013).

When patient groups cannot identify patients with experience of using the technology, they can present views of those living with the condition and current management processes. However, even with advance notice of deadlines, rapid review timelines can make it difficult for patient groups to obtain input from their members. Given the shortcomings of this approach, SHTG is planning to pilot a patient group submission form based on the HTAi (Chap. 6) and SMC templates.

SHTG has four Public Partners who are full members of SHTG. The number was increased recently from three to four to enable greater engagement of the Public Partners in topic selection and evidence assessment. Work is underway to explore whether Public Partners can present or lead a discussion of patient groups' views at meetings. Since July 2016, SHTG has held its meetings in public and invited relevant patient groups to observe. It is hoped that this will raise awareness and increase contributions to the SHTG process.

Given the challenges of gaining input from patients for non-medicine technologies, the timelines for certain topics may be increased to allow for qualitative synthesis to be incorporated.

27.5.2 Case Study of Patient Involvement in a Full HTA

A full HTA on antimicrobial wound dressings for chronic wounds used the HTBS model of patient involvement, with Public Partner participation in an expert advisory group and secondary and primary qualitative research (HIS 2015a). The aim and subquestions that guided the patient issues section of the HTA are presented in Box 27.1.

Box 27.1 Research Plan for Patient Issues Section

Aim

To explore and describe patients' experiences of chronic wounds and wound dressings (including antimicrobial wound dressings)

Sub-question

1. What is the burden of a wound on the daily lives of patients?
2. What are patients' current experiences of wound dressings?
3. What would patients like to see in the future with regard to the use of antimicrobial wound dressings?
4. What information on dressings is being communicated and shared by healthcare professionals with patients and their family/carers?
5. What are the views of patients and their carers on these dressings?
6. What factors affect access to antimicrobial wound dressings?

27.5.2.1 Methods

As an initial literature search using the Nederlands Huisartsen Genootschap filter for identifying patient issues for guideline development (Hielkema and Wessels 2014) did not identify much qualitative research, a specific search filter for qualitative material was developed in-house. Reference list searching and contact with authors identified more relevant studies than database searching.

No qualitative studies on antimicrobial wound dressings were identified, so the scope was broadened to studies of patients' experiences with chronic wounds and wound dressings for the qualitative synthesis. Primary research with people in Scotland who had experience of antimicrobial wound dressings was carried out. Analysis used the framework approach (Ritchie and Lewis 2009), which is recommended where data may be 'thin' (lacking in rich detail) and theory development may be limited. It provided a detailed and rigorous method of charting and summarising data and an audit trail leading directly back to the data supporting any findings. Data analysis was carried out in QSR NVivo10®. Box 27.2 outlines the process used in the qualitative evidence synthesis.

Box 27.2 Steps in the Framework Qualitative Evidence Synthesis

1. Familiarisation

Two researchers read all included studies with reference to the synthesis aims.

2. Identification of a thematic framework

One researcher examined all themes/findings from papers and developed an initial thematic framework. A second researcher independently reviewed the framework. Agreement was reached on amendments.

3. Indexing

One researcher systematically coded papers. The second researcher coded a subset of six high-quality papers. Results were contrasted and the framework further refined.

4. Charting

A matrix of findings for each theme by study was created by one researcher. The second researcher repeated the process for a subset of six studies and the results were contrasted. The subthemes were refined and differences in findings by wound type, study locations, etc. were examined.

5. Mapping and interpretation

Findings were refined and illustrative charts developed. The synthesis findings were incorporated into the HTA recommendations.

Box 27.3 Main Findings from the Patient Issues Section
The impact of chronic wounds on people's lives is considerable. The persistence, recurrence and symptoms of a chronic wound can have severe physical, psychological and social consequences.

- There is often a 'trial and error' approach to dressing selection, and this process can continue until the wound begins to heal. People may then credit a particular dressing type with healing their wound.
- Wound healing was usually the most important outcome to patients, but control of symptoms (in particular pain, odour and exudate) and prevention of infection and wound deterioration were also important outcomes. People often report wanting to 'try anything' to achieve these outcomes.
- The primary research revealed a positive view of antimicrobial wound dressings. People felt that they helped (or were helping) to heal their wound(s) and/or they helped with wound symptoms. One size did not fit all—an antimicrobial wound dressing that worked for one person may not work for another.
- Patients report that the extent and impact of pain from chronic wounds can be considerable. Reports of pain are not always acknowledged by healthcare professionals, and it seemed that pain frequently remains uncontrolled.
- People value care that they feel is personal and from healthcare professionals who they trust and who are persistent with treating their wounds even when wound healing is slow.
- The primary research indicated inconsistent access to antimicrobial wound dressings across healthcare settings. This led to frustration and inconvenience. This inconsistency is a likely influence on people who believed that the best treatments may be withheld from them because of costs.

27.5.2.2 Main Findings

The patient issues section provided rich information and findings about the experiences of patients with chronic wounds (Box 27.3).

27.5.2.3 HTA Recommendations

This work helped in the interpretation of all sections of the HTA. Incorporating patient views also provided triangulation, for example, patients' experiences and healthcare professionals' views highlighted the need for additional staff training around wound dressings and care for people with chronic wounds. It also directly informed recommendations 3 and 6 of the HTA (Box 27.4).

Box 27.4 HTA Recommendations Relating to Patient Issues
3: When selecting a dressing for people with chronic wounds alongside holistic clinical assessment, consider the factors of importance to the patient such as odour, pain/discomfort, leakage and mobility as well as healing.
6: A national patient leaflet should be developed, which can be used as an aid to support shared decision-making between patients with chronic wounds and healthcare professionals.

By investing in full and meaningful patient issues research, the HTA was able to produce valuable guidance for NHSScotland despite high levels of uncertainty in the clinical and cost-effectiveness sections.

27.5.2.4 Communication of HTA Results

Using guidance from the DECIDE project (DECIDE 2015, Fearns et al. 2016), a patient guide to the HTA (HIS 2015b) was developed in partnership with the HIS Public Partners.

27.6 Discussion

In SMC, patient involvement was conceived as an ongoing journey without a clear final destination. It had to be able to respond to opportunities as the policy environment, technology and people's attitudes changed (Nganasurian 2006). The hope was that involving patients would improve health services, increase acceptability of SMC decisions and reduce complaints about access to new medicines. Indeed, patient groups have shown that they can present clear examples and summaries about the impact of a condition and burden of treatments. This has provided useful context for SMC and helps committee members appraise the real value of a new treatment.

Some patient groups have expressed a desire to participate directly in SMC meetings to answer questions and clarify points of uncertainty. Some may also wish to present their own submissions rather than have them presented by Public Partners. However, it needs to be recognised that well-trained, experienced Public Partners can collate multiple submissions and draw committee's attention to those points most likely to influence decision-making. There is concern among some that persuasive presentations by stakeholders at SMC meetings might unduly influence the committee and impede members' abilities to make decisions based on the totality of the evidence, including the more complex clinical and cost-effectiveness analyses. Furthermore, there would be concerns about how to select a patient expert when several patient groups have an interest.

The scope of patient participation in SHTG has been much more limited. Although the challenges associated with identifying relevant patients for non-medicine technologies are clear, it is likely that the main explanation is the absence of dedicated patient involvement resources. SMC's high media profile and public petitions have led to an increase in resourcing specifically for patient involvement.

However, patient involvement has had an impact on SHTG HTAs. Patient group peer review comments on draft reports have contributed valuable information about organisational issues and unpublished surveys and audits. Furthermore, SHTG's wound dressing case study points to the value of qualitative research. In HTAs

where there is uncertainty around clinical and cost-effectiveness and/or in controversial or ethically uncertain areas, such research is especially likely to provide rich evidence and inform recommendations. Such analysis is resource intensive and requires investment in specific skills and/or collaboration with academia. Furthermore, research is needed to determine how best to incorporate qualitative patient evidence synthesis methods so that they can be used in the timeline of rapid HTA processes, which constitute the bulk of HTAs that are now undertaken by SHTG.

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KF is an independent consultant who undertakes unpaid work for HTA bodies and patient organisations but receives expenses to attend meetings. She also undertakes consultancy work for the pharmaceutical industry that is paid and may relate to HTA submissions and patient involvement strategies in drug development.

References

Bradbury I, Bonell E, Boynton J, Cummins E, Facey K, Iqbal K, et al. Positron emission tomography (PET) imaging in cancer management. Health technology assessment report 2. Health Technology Board for Scotland. 2002. http://www.healthcareimprovementscotland.org/previous_resources/hta_report/hta_2.aspx. Accessed 8 June 2016.

Bristol Royal Infirmary Inquiry. 2001. http://webarchive.nationalarchives.gov.uk/20090811143745/http:/www.bristol-inquiry.org.uk/final_report/the_report.pdf. Accessed 10 Nov 2016.

DECIDE. Developing and evaluating communication strategies to support informed decision and practice based on evidence. 2015. http://www.decide-collaboration.eu/. Accessed 13 June 2016.

Facey K, Cummins E, Macpherson K, Morris A, Reay L, Slattery J. Organisation of services for diabetic retinopathy screening. Health technology assessment report 1. Health Technology Board for Scotland. 2002. http://www.healthcareimprovementscotland.org/previous_resources/hta_report/hta_1.aspx. Accessed 8 June 2016.

Fearns N, Kelly J, Callaghan M, Graham K, Loudon K, Harbour R, et al. What do patients and the public know about clinical practice guidelines and what do they want from them? A qualitative study. BMC Health Services Res. 2016;16:74. doi:10.1186/s12913-016-1319-4.

HIS. Radical prostatectomy for localised prostate cancer: advice statement 004-13. 2013. http://www.healthcareimprovementscotland.org/our_work/technologies_and_medicines/earlier_advice_statements/advice_statement_004-13.aspx. Accessed 14 June 2016.

HIS. Antimicrobial wound dressings for chronic wounds: Health technology assessment report 13. Healthcare Improvement Scotland. 2015a. http://www.healthcareimprovementscotland.org/our_work/technologies_and_medicines/shtg_-_hta/hta13_antimicrobial_dressings.aspx. Accessed 10 June 2016.

HIS. Antimicrobial wound dressings for chronic wounds—Information for patients, their families and carers. Healthcare Improvement Scotland. Glasgow. 2015b. http://www.healthcareimprovementscotland.org/our_work/technologies_and_medicines/shtg_-_hta/hta13_antimicrobial_dressings.aspx. Accessed 31 May 2016.

HTBS. Guidance for patient, carer, and voluntary organisations on submissions of evidence to Health Technology Assessments. HTBS/0034/Jun 02. Glasgow. 2002.
Hielkema L, Wessels M. Power to the patient: the development of a literature search filter for patients' perspectives and preferences [conference proceedings]. 2014. http://www.iss.it/binary/eahi/cont/32_Hielkema_Full_text.pdf. Accessed 17 August 2016.
Nganasurian W. Patient and public involvement: five years on, October 2001–December 2006. Glasgow: Scottish Medicines Consortium; 2006.
Ritchie J, Lewis J. Qualitative research practice. London: Sage Publications; 2009.
Scottish Executive. Patient focus and public involvement. 2001. http://www.gov.scot/Resource/Doc/158744/0043087.pdf. Accessed 10 June 2016.
SMC. Patient and public involvement: two years on—a progress report. 2002.
SMC. Evaluation of the SMC's impact on and engagement with stakeholders—executive summary and full report. 2008. http://scottishmedicines.org.uk/files/SMC_engagement_with_stakeholders.pdf. Accessed 8 June 2016.
SMC. PACE (Patient & Clinician Engagement) overview document. 2016a. https://www.scottishmedicines.org.uk/files/PACE/PACE_Overview_Document_FINAL.pdf. Accessed 8 June 2016.
SMC. Patient Group Partners. 2016b. https://www.scottishmedicines.org.uk/Public_Involvement/Patient_group_partners. Accessed 10 June 2016.
SMC. Guidance to manufacturers for completion of Summary Information for Patient Groups. 2016c. https://www.scottishmedicines.org.uk/Submission_Process/Submission_guidance_and_forms/Templates-Guidance-for-Submission/SMC_Guidance_on_Summary_Information_for_Patient_Groups__V1_.pdf. Accessed 13 June 2016.
Slattery J, Chick J, Cochrane M, Craig J, Godfrey C, Kohli H, Macpherson K, Parrott S, Quinn S, Single A, Tochel C, Watson H. Prevention of relapse in alcohol dependence. Health technology assessment report 3. Health Technology Board for Scotland. 2003. http://www.healthcareimprovementscotland.org/previous_resources/hta_report/hta_3.aspx. Accessed 8 June 2016.
The Scottish Government. The healthcare quality strategy for NHSScotland. 2010. http://www.gov.scot/Resource/Doc/311667/0098354.pdf. Accessed 11 Nov 2016.
The Scottish Parliament. Key principles. 1999. http://www.scottish.parliament.uk/visitandlearn/Education/18651.aspx. Accessed 31 May 2016.
The Scottish Parliament. Patients Rights (Scotland) Act. 2011. http://www.legislation.gov.uk/asp/2011/5/contents. Accessed 11 Nov 2016.

Chapter 28
Sweden

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28.1 Introduction

This chapter presents patient involvement in HTA in Sweden. It details patient participation and patient-based evidence activities at the Swedish Agency for HTA and Assessment of Social Services (SBU) and the Swedish Dental and Pharmaceutical Benefits Agency (TLV). The chapter is written from the experience of the authors, who have worked with patient involvement at the respective agencies, and covers a number of different initiatives in this area, from the past, as well as current ones. SBU's experience shows how patients can be involved in a variety of ways throughout an HTA from being consulted with to being part of an advisory group or a reviewer for the HTA. TLV notes the challenges with involving patients in confidential medicine's reimbursement assessments, but shows how patients can be involved in development and broader ranging issues about medicines' policies. Both HTA bodies reflect on the importance of developing processes with patients and the need to evaluate impact.

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28.2 The Swedish Agency for Health Technology Assessment and Assessment of Social Services (SBU)

SBU is an independent national authority, tasked by the government with assessing healthcare interventions from a broad perspective, covering medical, economic, ethical and social aspects. Founded in 1987, it is one of the oldest HTA organisations in the world. Since July 2015, SBU has also been commissioned to assess interventions within the social services. SBU uses a range of mechanisms to involve patients in its HTA processes.

28.2.1 Patient Participation

Throughout SBU's history, individual patients and/or patient organisations have participated in different ways throughout the HTA process (SBU 2016a, pp. 42–43).

28.2.1.1 Patient Reference Group

Patient reference groups have shown themselves to be effective when SBU has multiple projects in the same area. One example is the series of five assessments SBU produced in the field of psychiatry between 2009 and 2013 (e.g. SBU 2012, 2013a, 2013b). In the early stages of these projects, a reference group was formed, consisting of two representatives from seven organisations of patients and/or their families.[1]

This group met four times throughout the project's life, with the project managers of the five SBU projects. The meetings were scheduled at the start, middle and end of the project, to coincide with important decision-making steps in the assessment process. Although the project status was briefly presented at each of the meetings, the main role of the SBU staff was to listen to and record the views of the reference group. At the first meeting, the patient representatives were asked to review the project protocols to ensure that the analyses would focus on outcomes that were relevant to patients and that issues important to patients had not been overlooked. Subsequent meetings focused on exploring patients' experiences, and the patient representatives were encouraged to provide feedback on preliminary results and formulating conclusions. They also discussed dissemination of findings and used the final meeting to evaluate the processes used with the reference group.

[1] Swedish Autism and Asperger Association, Swedish Depressive and Manic Depressive Association, Swedish National Partnership for Mental Health (NSPH), Swedish National Association Attention, Swedish Association for Social and Mental Health (RSMH), Swedish Association for Schizophrenia and Allied Disorders, Swedish Association for Obsessive Compulsive Disorder-Ananke.

28.2.1.2 Consultative Meetings

Some assessment projects have held consultative meetings with different stakeholders including patient organisations. For example, as part of a project on dietary treatment of obesity (SBU 2013c), the Swedish Association for Overweight People was invited to take part in a consultative meeting. They provided comments on the project protocol and the draft findings.

Sometimes, it is difficult to obtain a clear patient perspective, for instance, when the HTA concerns a field where there is no clear patient or user, e.g. prevention programmes offered to large populations where not all are at risk. One solution involves identifying alternative representatives. For example, the SBU project *Methods to Prevent Mental Ill-Health in Children* (SBU 2010a) sought comments on contents of the ethics chapter. SBU held a consultative meeting, and a group representing the users (students) was invited, but declined to participate. So, representatives from interested parties that were not represented at the external review were included, e.g. BRIS (Children's Rights in Society) and the Ombudsman for Children.

28.2.1.3 Multi-stakeholder Reference Groups

Sometimes, patient representatives participate in reference groups with representatives from other stakeholder groups. In a project considering patients' experiences and perceptions of professional care and support for self-harm (SBU 2015), the reference group included a representative from the Self Harm and Eating Disorder Organisation (SHEDO) as well as representatives from the three regional nodes of competence in the National Self-Injury Project initiated by the Swedish Association of Local Authorities and Regions (SALAR). This group helped formulate the right questions according to the PICO (Sect. 1.3.2) and SPICE[2] (Sect. 15.4) formats and provided feedback regarding the result findings and conclusions. The patient representative also helped in the dissemination of report findings and participated in media coverage and featured later in a film presenting the report results (SBU 2015).

28.2.1.4 Project Member

In the project on *Schizophrenia—Pharmacological Treatments, Patient Involvement and Organisation of Care* (SBU 2013a), one member of the project team was a patient and participated in the project group in this role. The others were health

[2] Before searching the literature for relevant qualitative studies for potential inclusion in an overview, it is usual to formulate a question and search policy according to the SPICE model, where S stands for setting, P for perspective, I for intervention, C for comparison and E for evaluation (the Joanna Briggs Institute 2008; A Booth 2004; SBU 2014, p. 18). SPICE is the equivalent of PICO, but used for qualitative studies.

professionals with different expertise relevant to the topic. In this case, the patient was able to explain, for example, the difference and relevance for the patient of the levels on the Positive and Negative Syndrome Scale which measures total symptoms as well as positive and negative symptoms respectively, i.e. the patient described the daily life implications of each level. This representative gave valuable input from the patient perspective on the project plan, its limitations and the questions raised. The input provided later on the scientific findings affected the way the results were presented.

28.2.1.5 Reviewer

Every SBU assessment report is both internally and externally reviewed. External reviewers are usually healthcare professionals who focus on the scientific quality of the manuscript. However, when patient participation in decision-making was assessed for psychosis/schizophrenia, ADHD[3] and autism spectrum disorders (SBU 2012, 2013a, 2013b), it was deemed necessary to also have the reports reviewed from a patient's perspective. Therefore, for the first time, SBU asked patients to provide feedback regarding the report relevance, focus, and comprehensiveness, from their perspective. The comments obtained from the patient reviewers clearly helped SBU produce a clearer and more relevant report.

28.2.1.6 Setting Priorities in Scientific Uncertainties

SBU maintains a database of scientific uncertainties in healthcare that have been identified by systematic assessments, in order to keep track of which methods require further research (SBU 2016b). Occasionally, a large number of uncertainties are identified for a condition, making prioritisation desirable. For instance, 39 uncertainties were identified in SBU's assessment of ADHD (SBU 2013b). SBU used a method for prioritisation inspired by the James Lind Alliance (James Lind Alliance 2016) to identify the ten most important uncertainties listed in that report from the perspectives of consumers and professionals.

The working group formed to prioritise uncertainties was composed of six people diagnosed with, or closely related to someone with ADHD, as well as seven representatives of the health, education and correctional services (psychologists, psychiatrist, primary care physician, corrective services officer, school counsellor and specialist pedagogue). The working group was not required to consider the feasibility of conducting research; meaning issues such as resources, research ethics and methodology were not taken into account.

Each member of the working group independently selected what he or she considered to be the ten most important uncertainties from the total of 39 listed in the

[3] Attention deficit hyperactivity disorder.

SBU report. The 20 items with the highest rankings were compiled for a workshop. At the workshop, initial discussion in small groups consisting of half consumers and half professionals resulted in a top ten list for each group. This was followed by a general discussion, in which the entire working group participated, to reach consensus on a final top ten list (Jacobson et al. 2016).

A similar current initiative at SBU is to prioritise uncertainties from the systematic map on prevention and treatment of maternal birth injuries following vaginal birth (SBU 2016c). It will be completed in 2017; more information can be found at SBU's website (http://www.sbu.se/en/ongoing-projects/).

28.2.1.7 Representation on SBU's Board

In 2012, SBU held a conference on patient collaboration. Approximately 100 patient organisations were invited to learn about SBU and discuss how SBU and patient organisations could work together. SBU collated the ideas, and a stronger commitment for patient and user involvement arose at SBU. As a result, following a request from the SBU management, the Swedish government appointed a patient representative[4] as a member of the SBU's Board of Directors.

28.2.1.8 Government Healthcare Agencies Collaboration

In 2012, the Swedish government started the PRIO project to prevent mental illness and improve health services for people with mental health issues. The project ran until the end of 2016. The National Board of Health and Welfare (NBHW) developed and tested a model for patient and relative engagement in mental health (Socialstyrelsen 2015, pp. 13–14). The NBHW wanted to do this in collaboration with other national bodies; thus, in 2014 they created a special group to strengthen the collaboration between patient/user organisations and four agencies, including SBU.[5] As a result, a model was developed (Fig. 28.1) for agency and patient and user organisation collaboration on a national level.

The model was piloted in September 2015 on the topic of mental health and pharmaceuticals. This was motivated by pharmaceutical treatment being one of the areas that concerned all patient organisations and all agencies and that is also included in the PRIO projects and in the quality registries.

[4] At time of publication, this was Stig Nyman, the Chair of the Swedish Disability Federation (Handikappsförbunden, HSO).

[5] Also included were National Partnership for Mental Health (NPMH or NSPH)—a network of organisations for patients, users and next of kin within the psychiatric field; NBHW; MPA; and the Public Health Agency of Sweden (FHM).

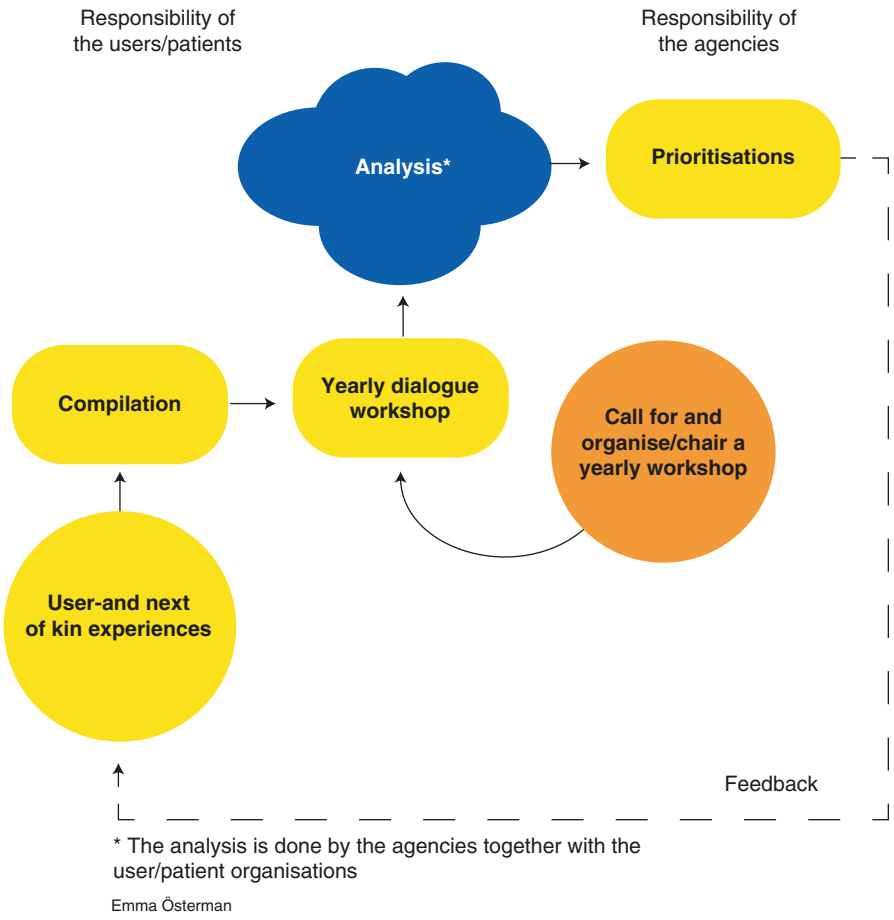

Fig. 28.1 A model of enhanced user participation

28.2.2 Impact and Future Developments

Each of the different forms of patient participation has given SBU beneficial additional information or insights. For example, the patient reference group for the projects in psychiatry insisted that we needed to make two separate reports – one for ADHD and one for autism spectrum disorders – rather than just one as planned. In other cases, patient feedback helped improve how report conclusions were formulated.

The strength of conducting systematic appraisals and assessments lies in the very thorough methodology. This line of thinking should also be applied to patient involvement. It is in the interest of HTA bodies to help develop robust processes to enable patient input to be systematically incorporated into their assessments. At SBU, a new strategic initiative is underway to incorporate the systematic and rigorous involvement of patients or users in the HTA project process. The project focuses

on developing activities, methods and tools, for both internal and external use, such as the translation and adjustment of the HTAi patient group submission templates (Sect. 6.4.2) for Swedish society.

The Government Healthcare Agencies Collaboration continues to develop and with some adjustments was tested again in September 2016, this time on the topic of mental health in the elderly.[6] All member agencies of the newly initiated Council for Knowledge-Based Policy[7] were invited to participate as well as the national coordinator in the field of mental health and the national investigator for a quality plan in elderly care. Although the analysis and assessment of that workshop are not yet complete, to some extent they have already been valuable for improving elderly and mental health in Sweden, as well as improving the model to create better conditions for collaborative work between patients, users and the governmental agencies. A working group on patient involvement has also recently been formed by the Council for Knowledge-Based Policy. This group is led by SBU and includes participants from all nine agencies.

28.2.3 Patient-Based Evidence

SBU has produced several HTAs that include patient-based evidence based on QES (Chap. 15), e.g. on topics like *Patient participation in decision-making in cases of psychosis/schizophrenia, ADHD and autism spectrum disorders* (SBU 2013a, 2013b, 2013c), loss of teeth and edentulousness (having no teeth) (SBU 2010b) and patients' experiences and perceptions of professional care and support for those who self-harm (SBU 2015). These reports focus on how people perceive and experience a condition, their health, their quality of life and/or their care or support. SBU has included patient experience in some or all of the above-mentioned areas, and sometimes the experiences of their families or relatives have been included. A systematic synthesis of experiences from published scientific studies makes up the body of evidence for these questions in the reports (in a separate chapter). These qualitative studies are assessed using similar methods to those applied to clinical or economic evidence. A systematic and structured assessment of their relevance, quality and methods of synthesis is conducted following the SBU Handbook on the Assessment of Methods in Healthcare (SBU 2014; Chap. 8). Examples of ongoing projects at SBU that aim to include a synthesis of studies using qualitative methodology can be found at SBU's website (http://www.sbu.se/en/ongoing-projects/).[8]

[6] The meeting was cohosted by SBU, Medicinal Product Agency (MPA), NBHW, FHM and NSPH.

[7] *The Council for Knowledge-Based Policy* has an advisory function and consists of nine governmental agencies: NBHW, MPA, SBU, TLV, the Swedish Research Council for Health, Working Life and Welfare (FORTE), the Public Health Agency, the Health and Social Care Inspectorate (IVO), the Swedish Agency for Participation and the Swedish eHealth Agency.

[8] For example, the benefit of surgery of arm fractures, family support and rehabilitation of children with alcohol spectrum disorder (FAS/FASD), parenting interventions for the prevention of physical or psychological child abuse or neglect, and interventions for unaccompanied asylum-seeking young people.

28.3 The Dental and Pharmaceutical Benefits Agency (TLV)

TLV is a central government agency that determines whether a pharmaceutical product, medical device or dental care procedure shall be subsidised by the state. TLV also determines retail margins for pharmacies in Sweden, regulates the substitution of medicines at pharmacies and supervises certain areas of the pharmaceutical market.

Since it was founded in 2002, TLV has involved patients. Its remit states that TLV is commissioned to cooperate with other government agencies and the NBHW and inform any stakeholder that is affected by its decisions. Collaboration with all external parties is an important and strategic area. TLV only undertakes processes to support patient participation. It does not undertake any research to generate patient-based evidence. Besides patient participation in its formal groups and boards, TLV can create processes to support patient participation according to the needs of specific areas of responsibility, as will be described later in this chapter.

28.3.1 Patient Representatives on Boards

TLV has two decision-making boards that decide on pricing and reimbursement in the Swedish healthcare system, the Board for Pharmaceutical Benefits and the Board for Dental Benefits. Board members are appointed by the Swedish government, usually for a period of 1–2 years.

Both decision-making boards have government-appointed representatives from patient and consumer organisations,[9] in order to ensure that patient aspects are taken into consideration when the boards decide on reimbursement and reference prices. These individuals do not represent individual organisations; they are responsible for taking a broad patient perspective in all decisions made.

TLV also has an advisory council, tasked to continuously review TLV's work and advise the Director General. There are also government-appointed representatives from patient and consumer organisations on TLV's advisory council.[10]

28.3.2 Dialogue Forum

The dialogue forum is a platform for informal discussions and information sharing between TLV and representatives of patient, pensioners and consumer organisations that meet twice a year. Patient organisations are invited if they receive an annual

[9] At time of publication, the patient representatives in the Board for Pharmaceutical Benefits are from the National Organization for Rare Disorders and the Asthma and Allergy Association. In the Board for Dental Benefits, one of the deputy members represents the Swedish consumer organisation.

[10] At time of publication, the representatives are from the Diabetes Association and from the Swedish National Pensioners' Organisation (PRO).

grant from government on the basis of fulfilling certain criteria. These include being formally established and of a certain size, active in several areas of society, democratically structured and politically and religiously independent.[11]

A committee with representatives from patient organisations and TLV creates a programme for the Dialogue Forum. The discussions should address ongoing strategic development projects at TLV and current trends and challenges in healthcare. During 2015, topics discussed included equal access to new medicines, medicines for rare conditions and consequences of not reimbursing prescribed medicines (TLV 2015b).

28.3.3 Development Projects

TLV's responsibility spans a wide, continuously changing area. Changes are often complex and involve several bodies' responsibilities, e.g. government agencies and other public and private organisations. One example of a situation that affects many bodies in the healthcare system is the new expedited or adaptive regulatory pathways that give early approval to innovative medicines and create a high degree of uncertainty in HTA. Another example is that of medicines for severe, rare conditions, which are difficult to study, and so the cost-effectiveness and budget impact are often substantially higher than the normally allowed levels in the reimbursement system. These new developments affect patients in many ways, so dialogue with the stakeholders, including patient organisations, is important to ensure a strong basis for decisions.

One common approach at TLV to include patients' perspectives in development projects is to form a reference group, composed of representatives from patient and consumer organisations. TLV has performed several development projects in various areas, where reference groups have been used. One example is when a reference group[12] was formed for TLV's review of how the ethical platform[13] applies to TLV's decisions and internal work, a project spanning over several years. As a result of this project, TLV made two changes to the way HTA is performed. First, for treatments that prolong life, the calculation methods should not discriminate against someone because they are not expected to work after treatment. Therefore, TLV no longer counts expected working years after treatment. Secondly, for treatments that affect quality of life, and simultaneously affect working, TLV should include results both with and without these effects. The application of this change will be formulated as there is experience of TLV's decisions (TLV 2015a).

[11] Between 2014 and 2015, approximately 60–70 organisations received these governmental grants.

[12] Consisting of representatives appointed by the Swedish Disability Federation (HSO), the PRO, the Swedish Association for Senior Citizens (SPF) and the Swedish Consumers' Association.

[13] In Sweden, all priorities in publicly funded healthcare are based on the ethical platform. The ethical platform was adopted by the parliament in 1997 and is based on three principles: the principle of human dignity, needs and solidarity and cost-effectiveness.

28.3.4 Individual Applications for Reimbursement or Recommendations

TLV believes that patient participation during HTA of medicines and consumables, as well as hospital medicines and medical devices, will help mutual information sharing and increase the quality of decisions or recommendations. TLV aims to obtain information from patients about, for example, the need for several medicines of a certain type or why a certain product or formulation is preferred by a specific patient group. Such information can be considered in the HTA and the final decision or recommendation. TLV also consults clinical experts during HTA.

28.3.4.1 Application for Prices and Subsidies for a New Product

When TLV assesses an application for reimbursement of a medicine or consumable, a company submission of evidence includes information that is confidential until TLV's decision is made. Thus, in order for TLV to engage in dialogue with any external party during assessment, the applicant company must waive their rights to confidentiality. As a result, dialogue with patient organisations about ongoing applications has historically been limited. However, when developing the value-based pricing project (TLV 2015c), some companies have waived their rights to confidentiality, allowing TLV to share information with representatives from patient organisations, e.g. the National Union of Hepatitis C (RHC) during the assessment of hepatitis C medicines (TLV 2015b).

28.3.4.2 Reviews of Reimbursed Products

When TLV initiates a review of medicines that are already in the reimbursement system, the confidentiality rule still applies to any material sent in to the agency. However, when these reviews are initiated by TLV, it is possible for TLV to have an open dialogue about which reviews are in process and the reason the review was initiated. This makes it possible for patient and consumer organisations to contribute their views and experiences earlier in the assessment process. For example, the Swedish Rheumatism Association was consulted when TLV initiated a review of the TNF-alpha medicines in 2015. The topics for discussion are specific for each review.

28.3.4.3 Assessment of Hospital Medicines and Medical Devices

TLV has performed HTAs of hospital medicines since 2009 and medical devices since 2012 (TLV 2013; TLV 2015a; TLV 2015b; TLV 2016). These HTAs are provided to the county councils to inform them in their investment decisions on

medicines and medical devices. Patient organisations have been invited to discuss the HTAs during development.[14]

28.3.5 Future Developments

Collaboration is a success factor for TLV in many ways. Dialogue with all stakeholders, including patient representatives, is essential to understand how TLV's decisions affect different areas of the healthcare system.

TLV aims to further develop dialogue with patients and users. Areas for improvement are paths of communication, meeting formats and the possibility to deepen dialogue in applications for reimbursement of new products (TLV 2015b, 2015c). This area will be continuously developed.

28.4 Discussion and Conclusion

Historical ambiguities in identifying the target audience for HTAs and similar knowledge-based documents and policies have further complicated patient involvement by making it difficult for both agencies and patient representatives to understand their respective roles (Läkartidningen 2016). If patients or users are seen as a target group or not affects the level of their involvement. SBU and TLV have always involved patients in their work and have developed various formal and informal ways of achieving this, but only SBU includes patient-based evidence as part of an HTA. The fact still stands, however, that Swedish HTA organisations continue to underuse patients and their experiences. SBU and TLV have used different approaches to involve patients in their work. These differences can be explained by the different commissions the respective agencies have been given by the Swedish government and to differences in the assessment processes each of the agencies has developed over time.

There are many reasons to involve patients in the HTA:

- Including patients' views and experiences
- Improving the content and quality of the reports and the decisions
- Sharing information
- Building trust for the decisions—increasing the understanding for HTAs and their results

[14] For example, during 2015, TLV invited the Swedish Heart and Lung Association, the Swedish Stroke Association and the HSO during the assessment of self-monitoring of warfarin treatment and the Swedish organisation for people with acquired brain injury, the Swedish Stroke Association and HSO during the assessment of thrombectomy.

The Swedish HTA agencies are responsible for providing evidence-based knowledge for other agencies (e.g. the NBHW who produce national guidelines), for doctors, other healthcare staff and prescribers (SBU) and for decisions on reimbursement (TLV). The conclusions they draw can have a major impact on patients' everyday lives. Early dialogue and information sharing are therefore of great importance in order to increase the quality of HTA and subsequently also increase the quality of guidelines. There are still some obstacles that need to be addressed in order to increase effectiveness and transparency. For TLV, the confidentiality rules and regulations set obstacles for open dialogue with patients, making it difficult or impossible for them to be consulted before the final decision is made public. TLV is currently exploring ways to further develop the dialogue with patient representatives in applications for reimbursement of new products (TLV 2015b; TLV 2015c).

The status of patients' perspectives might also be improved by ensuring that patient representatives get the same economic compensation as their professional counterparts, i.e. clinical experts. Currently this is done at SBU, but not yet at TLV.

The 2-year initiative at SBU to improve patient involvement that is underway at the time of writing this chapter is expected to increase the frequency and quality of patient input. The effort will help identify further obstacles and issues to be addressed, but will also lead to the routine production of reports that discuss outcomes and concerns that are important to patients. In the long run, by concentrating our efforts on assessing issues that are important to the user, and thus increasing their awareness of the evidence, we hope to improve the overall health of the Swedish population.

Finally, we highlight the need for collaboration across agencies on a national level. Initiatives such as the PRIO project and the Council for Knowledge-Based Policy should be encouraged to foster collaboration with respect to patient involvement. We would like to support the idea of a national patient panel, as proposed in the report by the Agency for Health and Care Services Analysis, as a method for gathering knowledge directly from patients (Vårdanalys 2015, p. 117f). Collaborative initiatives like these are well worth pursuing as the potential for mutual benefit is great and should be of high interest to agencies, county councils and patients.

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References

Booth A. Formulating answerable questions. In: Booth A, Brice A, editors. Evidence based practice for information professionals: a handbook. London: Facet Publishing; 2004.

Jacobson S, Östlund P, Wallgren L, Österberg M, Tranaeus S. Top ten research priorities for attention deficit/hyperactivity disorder treatment. Int J Technol Assess Health Care. 2016;32:1–8. doi:10.1017/S0266462316000179.

James Lind Alliance. http://www.jla.nihr.ac.uk/ (2016). Accessed 5 Sept 2016.

Joanna Briggs Institute. Reviewers' manual: 2008 edition. Adelaide: The Joanna Briggs Institute; 2008.
Läkartidningen. Kunskapsstöden är för många och för otydliga. 2016. 113;D6ZH. http://lakartidningen.se/Aktuellt/Nyheter/2016/07/Kunskapsstoden-ar-for-manga-och-for-otydliga/. Accessed 5 Sept 2016.
SBU. Methods to prevent mental ill-health in children. Stockholm: Swedish Council on Health Technology Assessment (SBU). 2010a. SBU Report No. 202 (in Swedish). http://www.sbu.se/en/publications/sbu-assesses/methods-to-prevent-mental-ill-health-in-children/. Accessed 5 Sept 2016.
SBU. Prosthetic rehabilitation of partially dentate or edentulous patients. Stockholm: Swedish Council on Health Technology Assessment (SBU). 2010b. SBU Report No. 204 (in Swedish). http://www.sbu.se/en/publications/sbu-assesses/prosthetic-rehabilitation-of-partially-dentate--or-edentulous-patients/. Accessed 5 Sept 2016.
SBU. Schizophrenia—pharmacological treatments, patient involvement and organization of care. Stockholm: Swedish Council on Health Technology Assessment (SBU). 2012. SBU report no 213a (in Swedish). http://www.sbu.se/213e. Accessed 5 Sept 2016.
SBU. Autismspektrumtillstånd—diagnostik och insatser, vårdens organisation och patientens delaktighet. En systematisk litteraturöversikt. Stockholm: Statens beredning för medicinsk utvärdering (SBU); 2013a. SBU-Rapport Nr. 215. ISBN 978-91-85413-54-6.
SBU. ADHD—diagnostics and treatment, organization of the health care and patient involvement. Stockholm: Swedish Council on Health Technology Assessment (SBU). 2013b. SBU Report No. 217 (in Swedish). http://www.sbu.se/adhd_2013e. Accessed 5 Sept 2016.
SBU. Dietary treatment of obesity. Stockholm: Swedish Council on Health Technology Assessment (SBU). 2013c. SBU Report No. 218 (in Swedish). http://www.sbu.se/218e. Accessed 5 Sept 2016.
SBU. Evaluation and synthesis of studies using qualitative methods of analysis. Stockholm: Swedish Agency for Health Technology Assessment and Assessment of Social Services (SBU); 2014.
SBU. Self-harm: patients' experiences and perceptions of professional care and support. Stockholm: Swedish Agency for Health Technology Assessment and Assessment of Social Services (SBU); 2015. SBU Report No. 2015-04 (in Swedish). http://www.sbu.se/201504e. Accessed 5 Sept 2016
SBU. Annual Report 2015, diarienummer EKO 2016/8 (in Swedish). 2016a. http://www.sbu.se/sv/om-sbu/arsredovisning-2015/. Accessed 5 Sept 2016.
SBU:s database on treatment uncertainties. 2016b. Stockholm, http://www.sbu.se/en/treatment-uncertainties/. Accessed 5 Sept 2016.
SBU. Behandling av förlossningsskador som uppkommit efter vaginal förlossning. En kartläggning av systematiska översikter. Stockholm: Statens Beredning för Medicinsk och Social Utvärdering (SBU); 2016c. SBU-Rapport No. 250 (in Swedish). http://www.sbu.se/250. Accessed 6 Nov 2016
Socialstyrelsen L. Statens beredning för medicinsk utvärdering, Folkhälsomyndigheten och Tandvårds—och läkemedelsförmånsverket. 2015. Samordnad statlig kunskapsstyrning inom området psykisk ohälsa—myndighetsgemensam delrapport av regeringsuppdrag (in Swedish). http://www.socialstyrelsen.se/publikationer2015/2015-1-48. Accessed 5 Sept 2016.
TLV: Ny tillämpning av etiska plattformen. 2015 (in Swedish). 2015a. http://www.tlv.se/lakemedel/halsoekonomi/Ny-tillampning-av-etiska-plattformen (Accessed 5 September 2016).
TLV Annual report 2015. 2015b. TLV reference number 3334/2015.
TLV: Utveckling värdebaserad prissättning, 2015 (in Swedish). 2015c. http://www.tlv.se/lakemedel/Utveckling-vardebaserad-prissattning/. Accessed 7 Nov 2016.
TLV: Brodin S, Kalin Y, Mörnefält J, Straubergs L. Delrapport–TLV:s regeringsuppdrag att genomföra hälsoekonomiska bedömningar av läkemedel som inte ingår i läkemedelsförmånerna, men som rekvireras till slutenvården, Tandvårds-och läkemedelsförmånsverket. 2013. TLV reference number: 2037/2011 (in Swedish).

TLV: Jacobi M, Kalin Y, Straubergs L. Klinikläkemedelsuppdraget—regeringsrapport 2015. 2015a. TLV reference number: 3556/2015 (in Swedish).
TLV: Blixt M, Södergård B, Hiort S, Nilsson C, Eckard N. Economic evaluation of medical devices. Final report. 2015b. TLV reference number: 187/2015.
TLV: Blixt M, Hiort S, Nilsson C, Södergård B. Hälsoekonomiska bedömningar av medicintekniska produkter. 2016. TLV reference number: 106/2016 (in Swedish).
Vårdanalys (The Swedish Agency for Health and Care Services Analysis) “Sjukt engagerad—en kartläggning av patient-och funktionshinderrörelsen”, report 4. 2015.

Chapter 29
Taiwan

Yen-Huei (Tony) Tarn

29.1 New Medicine Reimbursement Process in Taiwan

This chapter presents an overview of how decision-making processes for the reimbursement for new medicines have evolved in Taiwan over the last 20 years and how patient involvement has developed since it began in 2015. It outlines patient participation in the HTA programme that informs medicine reimbursement. It shows how Taiwan is encouraging patient participation in HTA via online submissions, participation in committee meetings and educational events and continues to develop its participation processes. The chapter highlights the challenges of patient participation in a setting where patient groups have little experience of engagement in policy. It also points to the need for guidance on using patient-based evidence in HTA and the potential value of resources that can be adapted to meet local needs and support HTA bodies developing patient involvement programmes.

Since Taiwan established its National Health Insurance programme in 1995, the Pharmaceutical Benefit Division has managed a decision-making process for new medicine (drug) reimbursement listing and pricing. The Drug Benefit Committee (DBC) was formed as an appraisal committee with 24 members who were physicians and pharmacists. However, due to the non-transparent process and lack of scientific evidence used to support decision-making, in 2007, the Department of Health (DoH) created an independent and unbiased HTA body. In 2008, the DoH set the budget and created the Division of Health Technology Assessment (HTA) under the Center for Drug Evaluation (CDE), which had been established 10 years earlier to conduct scientific reviews for regulatory purposes.

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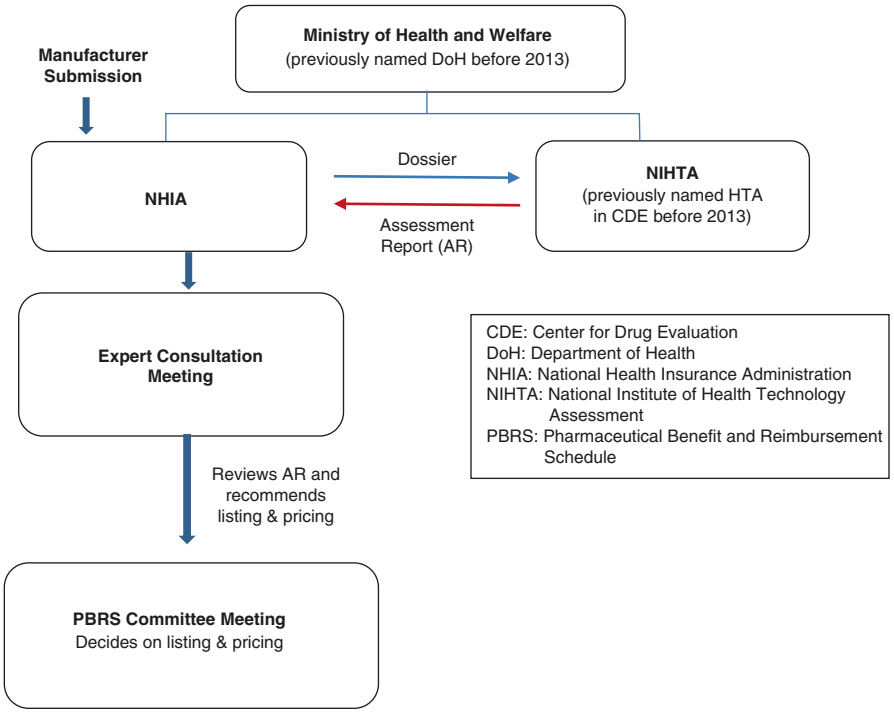

Fig. 29.1 The Decision-making process starting 2013 for new medicine listing and pricing in Taiwan (Ministry for Justice 2011)

In January 2008, the HTA function officially merged into the original process of new medicine reimbursement decision-making. The revised process after 2013 is presented in Fig. 29.1 (NHIA 2015).

When a dossier is prepared by a pharmaceutical company and submitted for new medicine reimbursement, the Pharmaceutical Benefit Division under the National Health Insurance Administration (NHIA) receives the case. If it is a new medicine application (new chemical entity, new route of administration or new combination), the application is sent to the HTA Division for assessment. The HTA Division conducts an independent systematic search for evidence—including comparative effectiveness and economic evaluation—and conducts its own budget impact analysis before writing an assessment report within 42 days. The assessment report is then sent to the NHIA where the expert consultation meeting (which replaced the original DBC in 2013) conducts the first appraisal and makes recommendations. The appraisal is then sent to the Pharmaceutical Benefit and Reimbursement Schedule (PBRS) Committee, which is composed of the NHIA members and representatives from various stakeholders (experts/scholars, the insured, employers and healthcare providers), to make the final decision on listing and pricing of the new medicines (Ministry for Justice 2013).

29.2 Developing Processes for Patient Group Participation

In 2013, Taiwan implemented the second-generation NHI Act (Ministry for Justice 2011), with the HTA process, the composition of PBRS Committee and transparency of process written in the law. The purpose was to have all stakeholders jointly make the decision. However, patient groups can be invited to attend but cannot vote in the PBRS Committee meetings. This is done for democratic and value judgement reasons, to learn more about patients' unmet medical needs and to provide a more comprehensive evidence base that incorporates patients' perspectives. However, due to the lack of process and guidance on how to prepare and present patient input to the PBRS Committee meeting, prior to March 2015, no representatives from patient groups were invited to the meeting.

Since 2013, the HTAi Interest Group for Patient and Citizen Involvement in HTA has created and actively promoted the patient group submission template for medicines and non-medicines HTA (HTAi 2014a) and published the HTAi *Values and Quality Standards for Patient and Citizen Involvement in HTA* (2014b). The template for medicines was translated into Chinese and the CADTH-pCODR patient group methods guide (2015) and *Patient Advocacy Group Input on a Drug Review* (2012) used in Canada were reviewed to create a Chinese version of the template and guide for patient advocacy groups by this chapter's author in 2014. These documents were sent to the Pharmaceutical Benefit Division of NHIA for consideration to be used in the new medicines reimbursement decision-making process in late 2014.

CDE has undertaken qualitative research to produce evidence about patients' perspectives and experiences for medical device HTAs, for example, in the assessment of cochlear implants. However, such research processes to obtain patient-based evidence have not been used with new medicines.

29.3 Establishing Mechanisms for Patient Input

In April 2015, the NHIA launched a web page which allows patients, caregivers and patient groups to submit their opinions about new medicines via the website (NHIA 2016). Fourteen days before the scheduled PBRS meeting, the inputs are summarised by the HTA division and sent to the PBRS meeting for consideration.

Patients, caregivers and patient groups can provide the following inputs:

1. In patients with this disease, what are the most uncomfortable symptoms or conditions that affect your day-to-day quality of life, and what symptoms or conditions cannot be controlled by your current therapy?
2. If you have not used the new medicine/medical device, what is your current therapy? How effective is it? Are there any side effects or uncontrolled symptoms?

3. If you have used the new medicine/medical device, is it more effective than previous therapies? Are there any side effects?
4. For the caregiver, are there significant improvements observed?

Although the web page is established, it has not been widely promoted. No one can confirm that the opinion given is from real patients or patient groups. The questions are simple without any probing examples (unlike the Chinese template) and may not really solicit the unmet medical needs of the patients. Patients who want to express their opinions may not know whether they need to answer all the questions or only answer one or two questions. That is, the current method is quite primitive, and there have been some calls to make improvements.

From May 2015 to May 2016, of the 88 new medicine submission cases, only three had patient input from the web page (personal communication with CDE). However, this is within a context of less than 60 patient groups in Taiwan (according to a list compiled by the International Research-Based Pharmaceutical Manufacturers Association). Most patient groups—approximately 70%—in Taiwan, are small. They tend to be associated with physicians and not independent. This creates challenges for involving them. In fact, in an educational program, only one patient group, the Breast Cancer Association, stated that they have the staff to complete the template and/or undertake training.

29.4 Patient Advocacy Group Education

In July 2014, the International Research-Based Pharmaceutical Manufacturers Association conducted the first half-day *Yes We Can Workshop* for patient advocacy groups. The concept of HTA and patient involvement in the new medicine reimbursement decision-making process was introduced to around 33 representatives from different patient groups.

In July 2015, the Taiwan Society for Pharmacoeconomics and Outcome Research (TaSPOR) held a half-day education programme for patient advocacy groups and pharmaceutical industries. The director of the Pharmaceutical Benefit Division of NHIA announced its mechanism for patient input. The author explained the importance of including patients' perspectives, where patients can participate in the HTA process, and how patient groups can collect patient input using a template. Other speakers provided information about qualitative methods used to provide robust evidence about patients' perspectives and how patient involvement is undertaken for quality of life measurement, burden of physical, mental health and daily living. Around 65 participants attended with 22 from patient advocacy groups. Participants raised the issues of staffing and the need for training about collecting and compiling information, sample size and representativeness of participants in the HTA process.

Another education event, held by the AmCham Medical Devices, Pharmaceutical, and Public Health Joint Committee in September 2015 focused on understanding,

accessing and building up partnership with patient advocacy groups. The President and CEO of the Canadian Organization for Rare Disorders, Dr. Durhane Wong-Rieger, gave a speech in English and people from the health technology authority, appraisal and agency attended and 13 patient advocacy group representatives attended.

29.5 Conclusions

Taiwan started patient involvement in the HTA decision-making process for medicine reimbursement in 2015. Much is to be learnt and the process needs to be monitored for appropriateness and validity. The template for patient groups to collect inputs is ready, and patient groups need to be encouraged to use it. More education programmes for patient groups need to be designed and implemented, especially about how to collect patient input, how to compile those inputs using the template and how to present this information in the appraisal committee meeting. Qualitative research processes used for other forms of HTA are interesting, but it is difficult to see how these can be used with the rapid process used for new medicines.

References

CADTH-pCODR. pan-Canadian oncology drug review: patient engagement guide. 2015. https://www.cadth.ca/sites/default/files/pcodr/pCODR%27s%20Drug%20Review%20Process/pcodr-patient-engagement-guide.pdf. Accessed 1 Mar 2016.

HTAi. HTAi Patient group submission template for medicines and non-medicines. 2014a. http://www.htai.org/interest-groups/patient-and-citizen-involvement/resources/for-patients-and-patient-groups.html. Accessed 1 Mar 2016.

HTAi. HTAi values and quality standards for patient & citizen involvement in HTA. 2014b. http://www.htai.org/interest-groups/patient-and-citizen-involvement/resources/for-patients-and-patient-groups.html. Accessed 1 Mar 2016.

Ministry for Justice. The National Health Insurance pharmaceutical benefits and reimbursement schedule. 2013. http://law.moj.gov.tw/Eng/LawClass/LawAll.aspx?PCode=L0060035 Accessed 1 Mar 2016.

Ministry for Justice. Article 41 and 42. In: National Health Insurance Act. 2011. http://law.moj.gov.tw/Eng/LawClass/LawAll.aspx?PCode=L0060001. Accessed 1 Mar 2016.

National Health Insurance Administration in Taiwan. About NHI. 2015. http://www.nhi.gov.tw/English/webdata/webdata.aspx?menu=11&menu_id=290&webdata_id=2974&WD_ID=290. Accessed 1 Mar 2016.

National Health Insurance Administration in Taiwan. NHIA webpage for patient, care giver and patient groups sharing opinions. (In Chinese). 2016. http://www.nhi.gov.tw/PatientShare/PatientShare.aspx. Accessed 1 Mar 2016.

pan-Canadian oncology drug review. pCODR patient advocacy group input on a drug review. 2012. https://www.cadth.ca/sites/default/files/pcodr/pCODR%27s%20Drug%20Review%20Process/pcodr-pad-input-on-drug-review.doc. Accessed 1 Mar 2016.

Chapter 30
USA: Comparative Effectiveness Research

Jaye Bea Smalley, Michelle Johnston-Fleece, Suzanne Schrandt, Lisa Stewart, and Sue Sheridan

30.1 Introduction

The USA has shifted away from HTA, which has the review of clinical trials at its core, to instigating a programme of clinical research that generates robust evidence about the value of health technologies in the US healthcare setting. The Patient-Centered Outcomes Research Institute (PCORI) was established as the US principal clinical comparative effectiveness research (CER) initiative in the landmark 2010 healthcare reform law, the Patient Protection and Affordable Care Act (PCORI 2010). PCORI's mission is to help people make informed healthcare decisions and to improve healthcare delivery and outcomes by producing and promoting high-integrity, evidence-based information that comes from research guided by patients, caregivers and the broader healthcare community. The Institute's work is guided by three strategic goals: increase information, speed implementation and influence research to be more patient centred (PCORI 2014). This chapter describes PCORI's processes, programmes, tools and evaluation efforts that are designed to involve patients and caregivers in the Institute's work and the funded research portfolio.

PCORI uses the term engagement to describe patient and stakeholder participation. Therefore, references to our tools, programmes, rubric and evaluation framework use the term engagement.

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30.2 Background

While the USA has had several HTA initiatives over the years, in general they have been fragmented and poorly coordinated. The unique multi-payer structure of the US healthcare system makes HTA a particularly politically charged issue with respect to objectivity and credibility, and funding mechanisms have changed over time due to policy decisions in the legislative and executive branches (Sullivan et al. 2009). The USA has shifted away from HTA and adapted CER as an approach that is unique to the US healthcare system and relevant to its interests. CER is focused on generating evidence that can be incorporated into decision-making at the point of care, whereas HTA tends to be more focused on synthesis and evaluation of evidence. CER became a focal issue in the 2009/2010 health reform debate. Its definition has taken on multiple dimensions and meanings and has not been clearly differentiated from either HTA or evidence-based medicine (Luce et al. 2010). Heightened political sensitivities challenge the structure, function and placement of any CER initiative in the USA (Wilensky and Developing 2006). Considering these sensitivities and the need for truly objective and credible evidence, PCORI's enabling legislation established the Institute as an independent not-for-profit, non-governmental entity with mandates to ensure transparency and participation of multiple stakeholders, with particular attention to the participation of patients and caregivers (PCORI 2010). PCORI characterizes patient-centred outcomes research (PCOR) as a type of CER. The PCORI's Board of Governors approved the following working definition of PCOR, which appears on its website: 'Patient-Centred Outcomes Research (PCOR) helps people and their caregivers communicate and make informed healthcare decisions, allowing their voices to be heard in assessing the value of healthcare options' (PCORI 2015).

The passage of the Patient Protection and Affordable Care Act in 2010 was a critical manoeuvre in US efforts to respond to increasing pressures on the healthcare system to improve the quality of care and population health and reduce the cost of quality care (AHRQ 2016). Patient-centred care has been identified as a critical aspect of healthcare improvement by The Institute of Medicine (IOM). The IOM defines patient-centred care as 'care that is respectful of and responsive to individual patient preferences, needs, and values and ensuring that patient values guide all clinical decisions' (IOM 2001 [48]). These reforms brought to centre stage the notion that it is imperative to bring patient and family voices to decisions about care, healthcare organizational design and governance and public policy (Carman et al. 2014). PCOR has solidified to address the decisional dilemmas patients and other healthcare stakeholders face due to a dearth of evidence about the effectiveness of diagnostic and treatment options given the heterogeneity of patient characteristics and individual preferences. Like other international efforts such as the Canadian Institute of Health Research's (CIHR) Strategy for Patient-Oriented Research (SPOR) initiative, PCORI has set out to develop support for the participation of a broad community of patients and caregivers in research. PCORI is prohibited in its authorizing legislation from funding any cost-effectiveness research or being involved in implementation activities that require advocacy for public policy change (CIHR 2014). This directive is a significant distinction from other HTA entities such as CIHR who involve patients all the way through implementation.

30.3 Rationale for Patient Participation

'PCORI funds research based on the belief that incorporating the patient perspective into healthcare research is inherently valuable and that including the end-user of research in the research process enhances usefulness and speeds the uptake of research into practice' (Frank et al. 2014 [1514]). This statement aligns with the instrumental goal for involving patients in HTA described in Part I of this book. PCORI identified similar principles to community-based participatory research (CBPR) when developing methodology standards to inform development of discrete methods standards for patient participation in design, implementation and dissemination of PCOR (PCORI 2012).

CBPR provides a framework to respond to health issues within a social and historical context while reducing mistrust of the people being studied, through collaboration and partnership between the researcher and community. The ultimate goal is for the community to own the results and use them to improve health outcomes and quality of life (Macaulay et al. 1999). Similarly, PCORI engages patients and caregivers with lived experience and their representative organizations to influence research to be patient-centred, relevant and useful and to establish trust and a sense of legitimacy in research findings to encourage successful uptake and use of results.

Patient participation is the process of enabling patients and caregivers to be partners in the research process, from the generation of the research question through dissemination. This partnership is distinct from research participation as subjects and characterized by active and meaningful participation among scientists, patients, caregivers and other healthcare stakeholders. Patient participation in research requires a thorough plan that may build upon existing partnerships.

30.4 Processes for Patient Participation

PCORI incorporates patient participation at both the organizational level and through its funding programmes. At the organizational level, there are numerous opportunities to engage patient and stakeholders in the work of the Institute. In addition to PCORI's Board of Governors, which includes members representing patients and caregivers, PCORI's authorizing legislation called for the establishment of advisory panels to provide recommendations and advice to the staff, board and methodology committee. These panels help review and prioritize research questions, provide technical and scientific expertise and provide other guidance around issues that may arise and are relevant to PCORI's mission and vision. All panels have members from the patient and caregiver community. Although it was not required by the legislation, PCORI established an advisory panel on patient engagement whose charge is to ensure the highest patient participation standards and a culture of patient-centredness in all aspects of PCORI's work.

30.4.1 Participation at the Organizational Level

30.4.1.1 Merit Review

Each merit review panel is composed of multiple stakeholders who provide unique perspectives to ensure that the research meets the Institute's funding criteria, which include patient-centredness and patient and stakeholder participation. PCORI works with reviewers to make sure all patients, caregivers and other stakeholders have the appropriate training to meaningfully participate in the merit review process. An early evaluation of the process found that patient reviewers brought different perspectives to the review process but that in-person discussion led to closer agreement among reviewer types (Fleurence et al. 2014).

30.4.1.2 Peer Review

To be sure that all PCORI research findings are critically appraised for scientific integrity as well as for adherence to PCORI's Methodology Standards (including patient-centredness and stakeholder participation) before results are released, PCORI is developing a peer review process as mandated in its authorizing legislation. The peer review process will be inclusive of patients and caregivers, and they will receive training to prepare them for their role as reviewers of all research final reports.

30.4.2 Participation in PCORI-Funded Research

Because patient participation in research is a novel funding requirement, most guidance on patient participation in research to date has been expressed through conceptual and theoretical models. Many stakeholders, eager to participate in PCOR, have asked for concrete guidance on this required element of PCORI's application process. In response, PCORI created the PCORI Engagement Rubric (Fig. 30.1), to serve as a framework for understanding how input from patient and stakeholder partners can be incorporated throughout the entire research process.

30.4.2.1 PCORI Engagement Rubric

The PCORI Engagement Rubric has become the cornerstone of PCORI's approach to participation in research and provides guidance to applicants, merit reviewers and staff on the development, evaluation and monitoring of participation plans for research. As a framework, the rubric points to opportunities for participation in research and shares real-world examples of promising practices from PCORI-funded research. The PCORI Engagement Principles promote reciprocal relationships, co-learning, partnerships, trust, transparency and honesty. Since its inception,

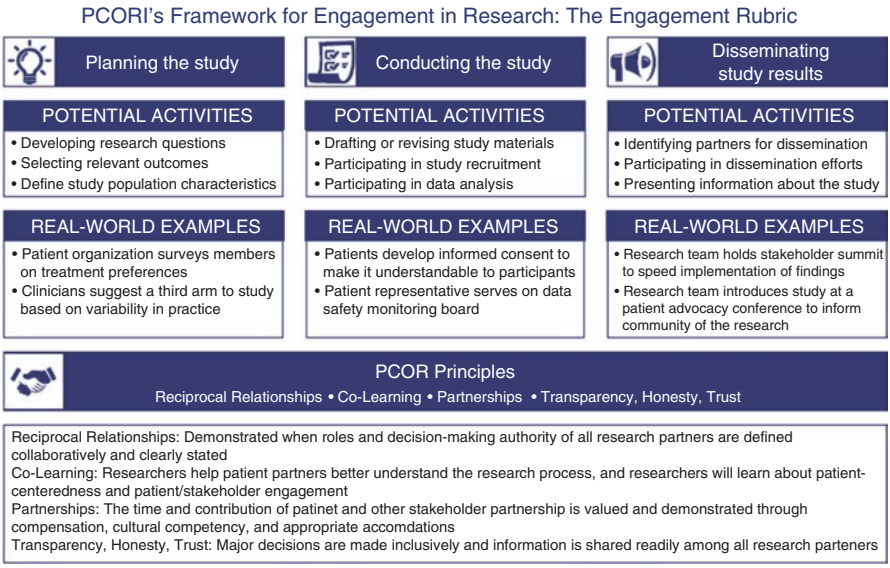

Fig. 30.1 The PCORI engagement rubric: a framework for engagement in research

the rubric has had several revisions to reflect new learnings gathered over time from PCORI's portfolio.

Example of Patient Participation in the Conduct of a Study The "Bringing Care to Patients: A Patient-centred Medical Home for Kidney Disease" study invited patients to participate in quarterly discussion groups at each dialysis centre to guide implementation of the intervention. The participants' input helps the research team plan, implement and evaluate the new care model being used in the study, particularly with respect to the availability of the primary care physician, pharmacist and health promoters during dialysis treatment. Their input also helps researchers to gain insight into patient and family member educational needs and interests (Cukor et al. 2016).

30.4.2.2 Engagement Officers

The creation of the PCORI Engagement Rubric highlighted the need for PCORI to monitor and learn from the patient participation practices employed by PCORI-funded researchers and led to the creation of the engagement officer role. Engagement officers work closely with the science programme to support funded research teams. They monitor research projects to ensure meaningful participation throughout the research project and gather promising patient participation practices and examples of impact of patient participation in research to share with the broader research community.

30.5 Evaluating Patient Participation

PCORI is a learning organization and has a dedicated programme that performs evaluation on all of its activities in order to further refine and enhance its processes and share lessons learned from our funded portfolio. PCORI's unique approach to evaluating participation is guided by a conceptual model and an evaluative framework. The conceptual model (Fig. 30.2) provides a structure for understanding the necessary elements of participation in research and a basis for evaluating the quality of participation and subsequent evaluation frameworks. The model describes key PCOR concepts and the relationships between them and includes principles for PCOR that are considered an ethical imperative to characterize research as PCOR (Frank et al. 2015).

PCORI's evaluation framework (Fig. 30.3) is characterized by the questions staff, patients and other stakeholders have asked about PCORI's novel approach to funding research and the ultimate impact that it may have on healthcare decision-making and outcomes. The questions are frequently refined because new questions arise as the Institute continues to learn about the impact of participation.

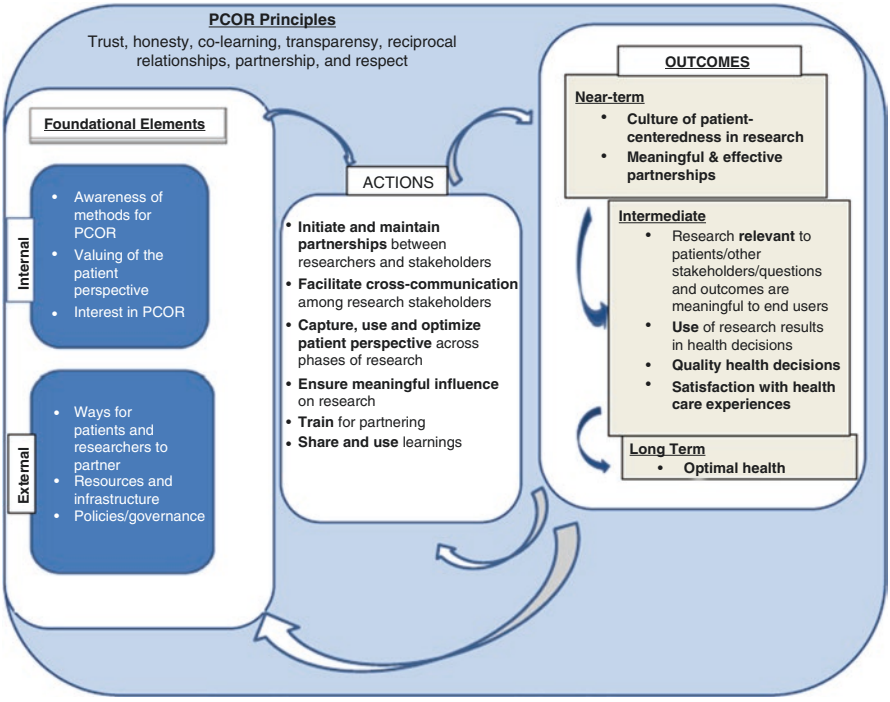

Fig. 30.2 Conceptual model of patient-centred outcomes research (Reproduced with permission from Frank et al. 2015)

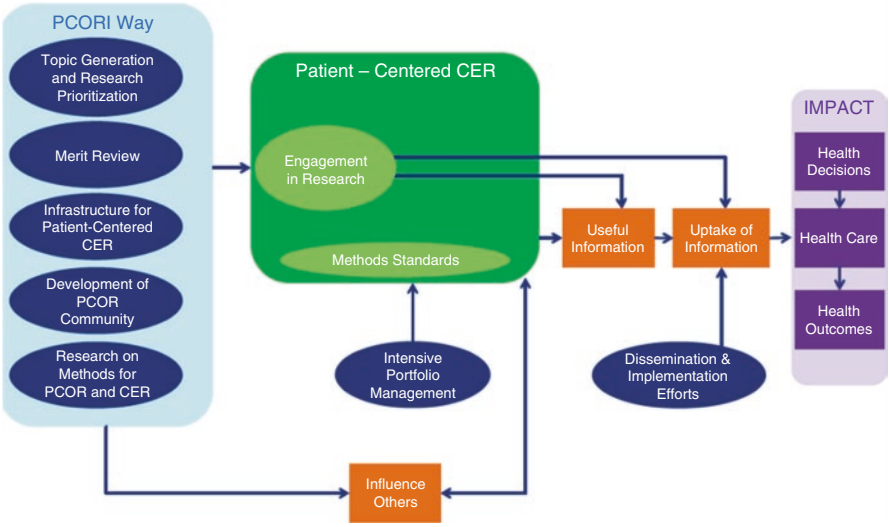

Fig. 30.3 Model for evaluating the overall impact of PCORI (Reproduced from PCORI evaluation framework 2.0 2015)

PCORI aims to better understand the relationship between levels of participation and changes to the research process, such as changes to study design and recruitment rates. To this end, PCORI developed tools to collect information from investigators receiving research awards and their patient and stakeholder partners, on participation in research (PCORI 2016a). PCORI uses an annual engagement report completed by the principal investigator of PCORI-funded studies and patient/stakeholder partners nominated by the principal investigators. Key evaluation domains include:

- Who, when and how of those involved
- Level of participation
- Influence of patient and stakeholder partners
- Impact of participation

The evaluation provides free text space for respondents to describe impact.

Example of Impact Statement Given in Evaluation from a Parent Participating in PCORI Pilot Projects Principal Investigator: "The parent stakeholders in our project shaped the research tremendously. Initially our principal investigator planned to ask families in our Pilot Project, 'If there was a cure for your child's chronic condition, what would it look like?' But participating families felt that had a negative tone. We actually had the experience of living with a child with a chronic condition. They did not. They didn't realize that when they asked the question that way it seemed like our child's chronic condition was all negative and we look at it in a positive light. We proposed, 'How does this disorder impact your child's life, and how would you like it to change?" (Brodt et al. 2015).

30.5.1 Learnings from Researchers

Forsythe et al. (2016) analysed patient and stakeholder participation in the 50 pilot projects funded by PCORI and identified early contributions and lessons learned. There was a 94% response rate from the principal investigators or their designees who self-reported on the types of stakeholders engaged, barriers and facilitators, lessons learned and contributions from patient and stakeholder participation.

30.5.2 Learnings from Patients

Key themes from a similar qualitative analysis of 257 patient and stakeholders in the first year of PCORI-funded research projects include several lessons learned from patient perspectives. Patients and stakeholders noted the importance of early participation. Patients also noted communication challenges such as managing power differentials, working with diverse groups and orienting to plain language. Many patients and stakeholders also reported impact beyond their PCORI research project, such as increased knowledge and skills about research (Forsythe and Johnston-Fleece 2015).

30.6 Capacity-Building and Future Directions

30.6.1 Capacity-Building Efforts

PCORI continues to advance the field of PCOR through a variety of programmes, participation in PCORI processes and work and, most of all, significant funding of PCOR. There is still much to learn from these efforts, and further work is needed to develop the PCOR community. Training is a challenge noted more broadly for organizing participation in HTA. While there is no substitute for the value that lived experience brings to the research setting, it is also important that these partners possess a general literacy around research terminology, methods and other aspects of the research enterprise. All researchers and partners may benefit from training on how to work on diverse teams. PCORI has capacity-building programmes that bolsters research partnerships and provides opportunities for PCOR-ready individuals or small groups of patients, caregivers or other stakeholders to come together with the goal of conducting research around a specific healthcare topic. For example, patients, caregivers and community-based organizations applied to receive funding to support partnership development with researchers and develop a shared research agenda (PCORI 2016b). PCORI considers the programme to be a learning laboratory of partnership development practices (McQuestion and Heckert 2015). Other awards are meant to foster the participation of more patients, caregivers, clinicians and other healthcare stakeholders into the research process. These awards are meant to better prepare communities for PCOR and serve as dissemination channels for

research findings by providing funding for implementing training and development initiatives, building knowledge, strengthening networks for dissemination and meeting and conference support. As an example, the University of Maryland partnered with the National Organization for Rare Disorders to develop PCOR training for rare disease advocates (PCORI 2016c).

30.6.2 Methods for Participation and PCOR

The development of methods to conduct PCOR is another strategic priority for PCORI. PCORI has a dedicated programme focused on improving methods for the conduct of PCOR. The field will benefit substantially from the development of rigorous methods to conduct participation, establish patient reported outcomes, prioritize research questions and conduct analyses that reflect the heterogeneity of real-world populations and individual response (McQuestion and Heckert 2015).

30.6.3 Future Considerations

The future of patient participation in research and HTA is both challenging and promising. We are beginning to see other key members of the research enterprise adopt patient-centred approaches to their processes as well as establish plans to involve patients. For example, the US FDA established a Patient Engagement Advisory Committee in 2016 (US FDA 2016a). They also held a series of 'patient focused drug development' meetings (US FDA 2016b). It is too early to determine the impact that these two initiatives may have on the agency, but it is encouraging to see an agency like FDA taking steps to advance patient participation.

It is still unknown where participation in the research process is most effective, the most successful approaches and the necessary levels of participation to impact various aspects of research that are important to patients such as outcomes. Many stakeholders are reluctant to involve patients without better understanding the return on investment for involving patients in the research process. Programmes conducting primary HTA research such as CIHR in Canada and NHS National Institute for Health Research in the UK also provide valuable information about patient participation arising from their long-standing experience in the field.

30.7 Conclusion

While further evidence and resources are needed to support a robust PCOR community, PCORI has driven the USA to move towards a more patient-centred research enterprise. Research is becoming increasingly focused on outcomes identified as

important to patients such as quality of life, functionality, symptoms and well-being (PCORI 2016d). We are also seeing the emergence of patient-driven research, where patients are custodians and generators of their healthcare data and serve as the catalyst and initiator of research. This is exemplified by PCORI's Patient-Powered Research Networks (PPRNs). These networks are characterized by communities of patients who have come together to develop and prioritize research agendas, develop and determine outcomes and leverage their electronic health record data, PRO data and patient-generated data from wearables and other technological devices in order to conduct these studies accordingly.

PCORI is committed to further developing the PCOR community and sharing learnings and advancements in patient participation. Early indications reveal a promising impact of patient participation in research. Many researchers report that patient participation has brought tremendous value to their work, so much so, that they now aim to conduct research that involves patients whenever possible. Also, patients and caregivers partnering in research report beneficial experiences beyond their work on the project such as increased knowledge about or engagement in their own health. (Forsythe and Johnston-Fleece 2015). PCORI faces similar challenges as other HTA's, particularly around leadership and developing new models and processes to organize a changing research enterprise. Like practitioners of HTAs, researchers will need to develop new skills, competencies and practices (Chap. 8).

PCORI aims to make a lasting impact in influencing more research to be patient centred and to ensure the PCORI vision that patients and clinicians have strong, evidence-based, patient-centred information to make decisions that reflect their desired health outcomes.

References

AHRQ. About the national quality strategy. (2016). http://www.ahrq.gov/workingforquality/about.htm#aims. Accessed 13 Aug 2016.

Brodt M, Norton M, Christine K, Kratchman A. So much more than a "pair of brown shoes": triumphs of patient and other stakeholder engagement in patient-centered outcomes research. Patient Exp J. 2015;2:43–9.

Carman KL, Dardess P, Maurer ME, Workman T, Ganachari D, Pathak-Sen E. A roadmap for patient and family engagement in healthcare practice and research. 2014. http://patientfamily-engagement.org/#sthash.lx5qltkN.dpuf. Accessed 13 Aug 2016.

CIHR. Strategy for patient-oriented research. 2014. http://cihr-irsc.gc.ca/e/documents/spor_framework-en.pdf. Accessed 13 Aug 2016.

Cukor D, Cohen LM, Cope EL, Ghahramani N, Hedayati SS, Hynes DM, et al. Patient and other stakeholder engagement in Patient-Centered Outcomes Research Institute funded studies of patients with kidney diseases. Clin J Am Soc Nephrol. 2016;11:1703–12.

Fleurence RL, Forsythe LP, Lauer M, Rotter J, Ioannidis JPA, Beal A, et al. Engaging patients and stakeholders in research proposal review: the patient-centered outcomes research institute. Ann Intern Med. 2014;2:122–30.

Forsythe L, Ellis L, Edmundson L, Sabharwal R, Rein A, Konopka K, et al. Patient and stakeholder engagement in the PCORI pilot projects: description and lessons learned. J Gen Intern Med. 2016;31:13–21.

Forsythe L, Johnston-Fleece M. Evaluating engagement in research: promising practices from PCORI's portfolio. 2015. http://www.pcori.org/sites/default/files/PCORI-Evaluating-Engagement-in-Research-Promising-Practices.pdf. Accessed 13 Aug 2016.

Frank L, Basch E, Selby JV. The PCORI Perspective on Patient-Centered Outcomes Research. 2014. http://www.sgim.org:8148/File%20Library/SGIM/Communities/Advocacy/Hill%20Day/jvp140114.pdf. Accessed 13 Aug 2016.

Frank L, Forsythe L, Ellis L, Schrandt S, Sheridan S, Gerson J, et al. Conceptual and practical foundations of patient engagement in research at the patient-centered outcomes research institute. Qual Life Res. 2015;24:1033–41.

IOM. Crossing the quality chasm: a new health system for the 21st century. 2001. http://iom.nationalacademies.org/Reports/2001/Crossing-the-Quality-Chasm-A-New-Health-System-for-the-21st-Century.aspx. Accessed 10 Apr 2016.

Luce BR, Drummond M, Jonsson B, Neumann PJ, Schwartz JS, Siebert U, et al. EBM, HTA, and CER: clearing the confusion. Milbank Q. 2010;88:256–76.

Macaulay AC, Commanda LE, Freeman WL, Gibson N, McCabe ML, Robbins CM, et al. Participatory research maximises community and lay involvement. Br Med J. 1999;7212:774–7.

McQuestion K, Heckert A. Developing methods to engage patients and other stakeholders research prioritization. In PCORI blog. 2015. http://www.pcori.org/blog/developing-methods-engage-patients-and-other-stakeholders-research-prioritization. Accessed 13 Aug 2016.

PCORI. PCORI Authorizing legislation. 2010. http://www.pcori.org/sites/default/files/PCORI_Authorizing_Legislation.pdf. Accessed 14 Aug 2016.

PCORI. Workshop on incorporating the patient perspective into patient-centered outcomes research. 2012. http://www.pcori.org/sites/default/files/Workshop-on-Incorporating-the-Patient-Perspective-into-PCOR1.pdf. Accessed 14 Aug 2016.

PCORI. About us. 2014. http://www.pcori.org/about-us. Accessed 30 Sept 2016.

PCORI. Definition of PCOR. 2015. http://www.pcori.org/assets/March-5-Definition-of-PCOR11.pdf. Accessed 13 Aug 2016.

PCORI. How we evaluate key aspects of our work. 2016b http://www.pcori.org/research-results/evaluating-our-work/how-we-evaluate-key-aspects-our-work. Accessed 13 Aug 2016.

PCORI. Partnering to advance pediatric health equity through research engagement. In Research and Results. 2016c. http://www.pcori.org/research-results/2016/partnering-advance-pediatric-health-equity-through-research-engagement. Accessed 13 Aug 2016.

PCORI. PCOR training: a program for rare disease patient advocates. In: Research and results. 2016d. http://www.pcori.org/research-results/2015/pcor-training-program-rare-disease--patient-advocates. Accessed 14 Aug 2016.

PCORI. Planning our evaluation, reporting the results. In: Research and results. (2016a) http://www.pcori.org/research-results/evaluating-our-work/planning-our-evaluation-reporting-results. Accessed 14 Aug 2016.

Sullivan SD, Watkins J, Sweet B, Ramsey SD. Health technology assessment in health-care decisions in the United States. Value Health. 2009;12:S39–44.

Wilensky GR, Developing A. Center for comparative effectiveness information. Health Aff. 2006;25:w572–85.

U.S. FDA. (2016a). http://www.fda.gov/AdvisoryCommittees/CommitteesMeetingMaterials/PatientEngagementAdvisoryCommittee/default.htm. Accessed 14 Aug 2016.

U.S. FDA. (2016b). http://www.fda.gov/forindustry/userfees/prescriptiondruguserfee/ucm368342.htm. Accessed 14 Aug 2016.

Chapter 31
Discussion of Approaches in Different Countries

David Hailey

31.1 Introduction

This chapter reviews the developments and challenges in patient involvement in HTA presented by different countries in Part III. The different approaches presented provide interesting and detailed updates to information previously obtained in surveys by HTAi, INAHTA and other groups (Chap. 5). Most of the chapters describe processes for patient involvement in national HTA programs. The Canadian chapter also covers the different approaches to patient involvement that have been taken in the provincial HTA programs. International perspectives are taken in the chapter on the HTA Core Model, and Chap. 30, from PCORI, deals with initiatives in CER in the USA. This chapter contrasts the developments in the application or introduction of patient involvement in HTA in the countries and their relation to the political context. The main focus of the HTA programs is in providing advice to health ministries or health services. It is clear that the approaches used by the HTA programs to gain patient input or undertake research into patient aspects vary and are still evolving. Challenges include pressure on patient organisations and HTA programs through demands on their time and resources. Further work is needed to document and report the effects of patient involvement on the preparation of HTA reports and their recommendations to ensure more effective patient involvement in HTA in the future.

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31.2 Organisation and Political Framework

Part III shows that the organisational structures and political context of HTA programs and the decision makers they are seeking to inform are sometimes quite complex. They typically include committees or councils with some relationship to the health ministry or health service provider, sometimes within such organisations. Support for the committees, including preparation of HTA reports, may come from independent organisations, from university-based and other contractors or from within—house. In all of the countries covered, there have been changes in organisations over time, reflecting political and governance decisions. These can have consequences for the operation of HTA programs and for patient involvement. Some of the programs have had an initial focus on assessment of medicines or devices, though this is starting to widen. Most undertake both medicine and non-medicine assessments. Chapter 28 indicates the wide ambit of HTA activities in Sweden, with patient involvement in the assessment of many types of technologies and services. In Germany and Taiwan (Chaps. 25 and 29), patient involvement in HTA is specified in legislation, and it has been a strong policy focus of the Scottish Parliament (Chap. 27).

31.3 Approaches to Patient Participation

In general, the various programs follow the approaches detailed in Chap. 6 though the level of involvement varies. All of the HTA programs covered in this part offer opportunities for patient organisations to provide input, and some allow individuals to provide comments to committees or multi-stakeholder advisory groups. In some cases, these processes are still being developed. Also, submissions may be considered without feedback to the patient organisation or individual, so that there is no interactive component in the process. There is increasing inclusion of patient representatives on committees (Chap. 21) typically through nomination by patient organisations. In Germany, patients and organisations are involved in the entire process including preparation of HTA reports but are not permitted to vote. Committees may include both topic-related patient representatives who are knowledgeable about the technology under consideration and permanent representatives with methodological or general expertise.

Most of the HTA bodies provide details of training and support for patient organisations and individual patients and caregivers. This includes written materials aimed at patient organisations and plain language summaries for a broader audience. Some examples are provided of summaries and versions of HTA reports that are intended to be easily understood by patients (Chaps. 20, 23, 28 and 30). Support from PCORI (Chap. 30) includes availability of an engagement rubric as a framework for how patient involvement can be incorporated through the research process. Development of this is continuing. Some support is more extensive including legal and methodological advice (Chap. 25) and availability of engagement officers to assist projects (Chaps. 21 and 30).

Communication of HTA findings to patients and caregivers is generally good, with approaches still emerging in some of the more recently established programs. The impression is that this aspect has improved over the last few years with appropriate transparency in assessments. Some limitations remain, as with the arrangements for PBAC decisions mentioned in Chap. 19, though these seem to have improved since an earlier review that considered the level of disclosure to be poor compared with the requirements of other regulatory processes (Productivity Commission 2005).

31.4 Research into Patient Aspects

Use of QES by some programs is discussed (Chaps. 21, 22, 27 and 28) with informative examples of its application. The need for qualitative methods is noted in Chap. 29. Whilst use of the analytical hierarchy process is described in Chap. 25, no country appears to have used discrete choice experiments.

All the methods presented in Part II may be options for the future, giving opportunities to broaden assessment approaches and patient involvement. Some of the methods are time-consuming and expensive and may require specific research expertise. That suggests the need for selection of appropriate methods for specific HTAs, with a clear understanding of why an approach is being used and of its expected contribution to the decision-making process.

In Chap. 24, details on the development and structure of the Patient and Social Aspects domain of the EUnetHTA Core Model® give a useful account of issues to be considered and relationships to other parts of the HTA process. The HTA Core Model® was helpful to the Italian program in overcoming diffidence towards the use of qualitative methods (Chap. 26).

Use of focus groups and interviews is referred to in Chaps. 21 and 22 as approaches for getting descriptive information rather than full qualitative research, when available secondary data are not sufficient. Recruitment of suitable participants may be difficult and time-consuming, possibly causing delays to HTA projects.

31.5 Challenges and Limitations

31.5.1 Input from Patient Representatives

Recruitment and training of patient representatives on committees and obtaining their appropriate input to discussion continue to be demanding. To their credit, most of the programs are active in seeking improvements in these areas. Examples include a focus on finding ways to lea**r**n from patients, rather than adhering to fixed processes (Chap. 27), a project on inclusion of patient involvement in a systematic and rigorous way (Chap. 28), evolution of approaches to involve and encourage patient participation (Chaps. 23 and 27) and pilot studies on patient preferences (Chap. 25).

31.5.2 Resources and Timelines

Including patient involvement in an HTA program is resource intensive. Suitable support must be provided for patient representatives to participate in committees and other areas of involvement. Organisational requirements for patient involvement place additional demands on HTA programs. Expertise in some of the research methods used to obtain information about patients' preferences and perspectives can be limited within an HTA body and may need to be provided by external contractors. HTA programs can come under scrutiny by government organisations seeking budget savings. Committing limited funding to patient involvement may be difficult for programs, and government support for some initiatives can be uncertain. Chapter 19 mentions discontinuation of funding for patient impact statements that included details of how a condition affected patients' daily lives and the impact on caregivers.

As discussed in Chap. 27, patient involvement may become challenging with rapid HTAs, with pressure both on patient organisations and HTA program staff. A need is seen to develop rapid quality evidence synthesis methods for use in rapid evidence reviews which form a major part of the Scottish HTA program.

31.5.3 Measuring Outcomes

The outcomes of patient involvement in HTA are of considerable interest but often difficult to specify or quantify. Such details are needed to refine approaches taken by HTA bodies, to provide input to reviews of the HTA programs and requests for funding and for feedback to patient organisations. The extent of patient involvement can be documented through recording receipt of communications from patients and caregivers, for example, through use of patient input templates, as discussed in Chap. 21. Communication by the HTA program with patient organisations and other stakeholders can also be recorded. But beyond this basic level, there are issues of to what extent patient involvement has influenced the preparation of HTAs and the decisions that they inform.

At one end of the spectrum, there are cases where patient involvement has clearly had a major influence on the scope or organisation of an HTA. Several excellent examples are given in the chapters from the HTA programs and are worth documenting and publicising. But the influence of patient involvement on some assessments will be less obvious. Chapter 21 notes it is challenging to track the input of information collected through templates on HTAs or deliberations, and Chap. 25 points out barriers to evaluation of patient participation in committee meetings. Routinely including a section on patient-related issues in HTA reports may be helpful, as may occasional reviews of the HTA program (Chaps. 21 and 30). PCORI (Chap. 30) describes an evaluative framework characterised by questions from patients and other stakeholders on the approach to patient outcomes research and effects on decision makers and outcomes.

31.6 Patient Involvement in HTA-Based Decision-Making

As noted in Chap. 1, the new WHO directive supporting the use of HTA to inform decisions about universal health coverage could have significant impact on developing countries with scarce resources. HTA is promoted as a systematic and transparent framework to inform decision-making. There would be general agreement in the HTA community with this, but also recognition that such a framework will still not lead to consistency in the transparency of the decision-making processes themselves. Transparency in decision-making depends on the political constructs of the countries and regions and is part of the political process both for government and non-government decision makers. Decision makers should not ignore HTA findings and recommendations, but they do not have to accept them. Typically, HTA will be one of several inputs to the decision-making process. Other factors considered by decision makers include political views, existing policies, administrative feasibility, timing and equity (Ross 1995). Also, HTA is not confined to evaluation of clinical and cost-effectiveness. Other areas frequently considered by HTA in provision of advice to decision makers include safety, burden of disease, budget impact and social issues. In principle, there could be a major role for patient involvement in poor countries through providing input on issues with the safety and effectiveness of available health technologies and services.

31.7 Future Directions

As discussed in Chap. 5, patient involvement initiatives in 2005 included preparation of a discussion paper by INAHTA and a survey of its member agencies. Various challenges associated with patient involvement in HTA were identified, and some, as presented in Chap. 5, continue to be present (Facey and Stafinski 2015). Difficulties included issues with time constraints when projects were carried out in response to urgent requests and the need of resources for identification and training of suitable organisations and individuals (Hailey 2005). A follow-up survey of INAHTA agencies suggested that the level of patient involvement remained relatively limited with little inclusion of consumer perspectives in assessment reports (Hailey et al. 2013).

The experiences of the programs in different countries presented in Part III show the considerable progress in patient involvement in HTA that has been made over the last decade following the earlier initiatives. As well as the substantial efforts of the HTA community, there is growing acceptance within bureaucracies that patient involvement has a valuable place in decision-making. There is also, in general, increased sensitivity at the political level to patients' rights and perspectives and to pressures via social media.

However, whilst there have been substantial advances in many countries, patient involvement in HTA is still a work in progress. The arrangements for patient

involvement and interaction with HTA programs are still evolving. Projects or plans for the programs include broader involvement with patient organisations with further training and support. Several programs are concerned with better analysis and documentation of patient input to assessments and recommendations. As noted in Chap. 21, better links are needed between patient input to HTA and for what and how it is utilised. Further development of qualitative methods and methods for qualitative evidence synthesis is also seen as necessary.

Changes within ministries and in government policies have consequences for HTA, including patient involvement. In some countries, these changes have been rather frequent, and the associated administrative arrangements can be complex. It is not unknown for there to be pressure on bodies to provide faster HTA findings, with limited resources (Chap. 19). With HTA bodies that are external to the health ministry, difficulties may be compounded by delays within bureaucracies in responding to draft reports or providing confirmation of support for an assessment. Political sensitivities and demands on HTA bodies are touched on in several chapters (Chaps. 19, 21, 25, 26 and 27).

Effective patient involvement in HTA depends, among other things, on informed and appropriate input from patient organisations. There will be limits to what can be done from their usual resources, and this could become an increasing difficulty as inclusion of patient involvement in HTA projects widens. Limitations in support for patient organisations are mentioned in Chaps. 22, 25, 27 and 28. Sources of funding may need to be identified to ensure that patient organisations are a sustainable resource for HTA. Several chapters raise the issue of deciding which technologies should have patient involvement in their assessments, but there is also the issue of which questions or aspects of a technology require such input. For example, assessments considering relatively minor changes to devices might or might not warrant patient input.

With these various pressures, HTA programs may find themselves between a rock and a hard place. Inclusion of patient involvement in rapid HTAs seems particularly demanding of both patient organisations and HTA programs. As indicated in Chaps. 21 and 27, flexible approaches will be needed as appropriate for each HTA program and assessment topic, taking account of both timelines and available resources. HTA programs will also have to further develop their ability to routinely record and report on the contribution of patient involvement activities to the preparation of assessments and of advice to decision makers.

References

Australian Government Productivity Commission. Impacts of advances in medical technology in Australia. Research Report. Melbourne. 2005. http://www.pc.gov.au/inquiries/completed/medical-technology. Accessed 12 Oct 2016.

Facey K, Stafinski T. Tools to support patient involvement in Health Technology Assessment. In: G-I-N public toolkit: patient and public involvement in guidelines. 2nd ed. Pitlochry: Guidelines International Network; 2015. p. 141–159.

Hailey D. Consumer involvement in Health Technology Assessment. Edmonton: Alberta Heritage Foundation for Medical Research, HTA Initiatives #21. 2005. http://www.ihe.ca/advanced-search/consumer-involvement-in-health-technology-assessment. Accessed 20 Sept 2016.

Hailey D, Werkö S, Bakri R, et al. Involvement of consumers in Health Technology Assessment activities by INAHTA agencies. Int J Technol Assess Health Care. 2013;29:79–83.

Ross J. The use of economic evaluation in health care: Australian decision makers' perceptions. Health Policy. 1995;31:103–10.

Chapter 32
Discussion: Patient Participation in HTA—Evidence of Real Change?

Durhane Wong-Rieger

32.1 Introduction

Only a few years ago, it was unlikely we would find enough evidence of patient involvement in HTA to fill a chapter, let alone a whole book. Clearly, progress has been made, and undoubtedly there will be much more to come; nevertheless, it may be timely to ask: How much real change has taken place in HTA bodies and, as importantly, what has been the impact? This chapter explores this question by considering the evidence from the various countries and/or regions presented in Part III. My own experience, working with patient organizations in Canada and internationally via IAPO, allows me to critically review processes from the perspective of patient advocates who are keen to be involved in HTA processes. First, there are systematic similarities and differences in the ways in which patients are participating in HTA reflecting ideological, developmental, and cultural factors. Second, the goals of stakeholders for patient participation vary both within a healthcare system and across systems, with some goals naturally more conducive to patient involvement than others. Third, there are conditions that support or detract from patient participation in HTA, and examination suggests some best practices that we can build upon.

32.2 Evidence of Increasing Patient Involvement

As laid out for each country or region in the preceding chapters and succinctly summarized by Hailey in Chap. 31, the number of HTA institutions reporting patient involvement has increased, especially over the past few years. However, the scope

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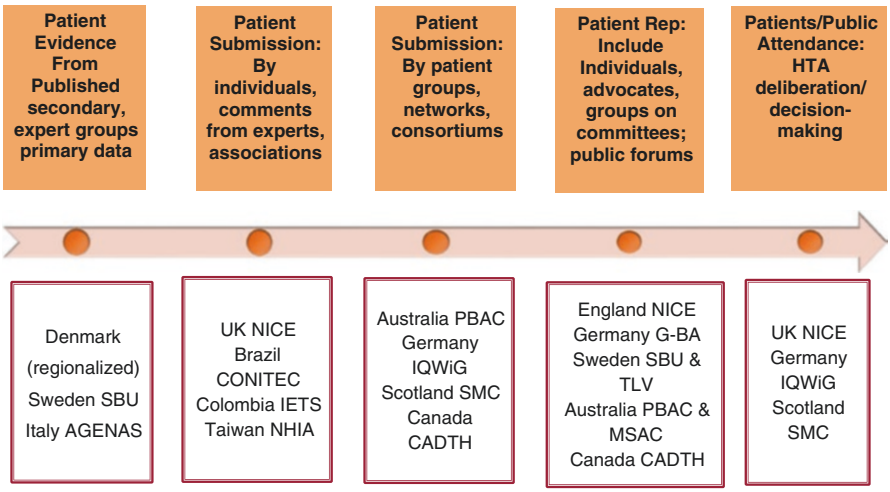

Fig. 32.1 Patient involvement in HTA: country differences

and nature of the involvement are quite varied. Nevertheless, while we are clearly in an era of exploration and experimentation, the discussions in this book suggest there may be convergence toward similar if not yet best practices.

HTA assessments initially included only patient-based evidence that met "scientific" standards of collection, measurement, and analysis primarily quality of life measures as part of clinical trials. The patient and caregiver were not active partners but subjects in the production of patient-based evidence responding to questionnaires including validated instruments.

As patients mounted, pressure to have an active voice, HTA institutions have responded in various ways. As shown in Fig. 32.1, three of the HTA systems presented here do not have a process for direct patient input, incorporating only published secondary source data or primary data collected by the HTA body from expert or reference groups. Nevertheless, it appears that all are interested in moving toward means for direct patient input, whether at a national level (Sweden, Italy) or regionalized HTA level (Denmark) (Chaps. 22, 26, and 28).

Some HTA institutions have opened a pathway for individual patients and caregivers to provide direct input on a specific technology under review, which considerably lowers the barriers to involvement. Being provided questions to answer and a template for preparing the submission, patients and patient groups may share the experience of living with a condition or the consequence of not having a specific therapy with little or no training in HTA. Taiwan has implemented a process for patient and caregiver testimony, albeit limited to a 300-character written submission to the appraisal committee (Chap. 29). Brazil does not solicit input prior to the appraisal but does publish proposed HTA recommendations on their website for patient and public commentary (Chap. 20). Perhaps not coincidentally, these jurisdictions assure patients (public) the legal right to participate in health policy and/or healthcare decision-making.

Institutions in some countries (Germany, England/Wales, Scotland, Canada, Australia) receive patient experiences and opinion through patient groups, networks, or consortiums (Chaps. 19, 21, 23, 25, and 27). This pathway has several advantages, including reducing the number of submissions, acquiring summary experience and opinion, and improving the quality of input. Because most participating groups are designated, registered, or otherwise solicited, the HTA body has the opportunity to improve the quality of the input by structuring the format and supporting education and/or training.

Appointment of patients and/or patient representatives to appraisal and decision-making committees represents a higher level of participation and, depending on the country, has proceeded, succeeded, and coincided with the development of a patient group submission pathway. Interestingly, patient representative roles vary across institutions, as do their qualifications. While all appointees are required to represent patients *writ large*, in some committees they are technically public representatives (Canada) or lay members (England/Wales) and in others may be nominated by the patient organizations (Germany, Australia). The scope and level of participation also differ, from providing comment only on specific technology assessments to involvement in therapeutic category reviews to health services redesign.

There are three countries where the HTA deliberation process is open to patients and/or the public (England/Wales, Scotland, and Germany) although the format varies.

32.3 Goals and Impact of Patient Involvement

It is difficult to determine whether patient involvement has had a meaningful impact on HTA outcomes as outlined in Chap. 16. Beyond the ethics and politics of patient participation, it was suggested that patient-lived experience with the condition and/or therapy would enhance the quality of the assessment and by extension lead to recommendations that were individually more appropriate and in aggregate the best allocation of health resources. However, measuring the impact on quality of recommendations may be difficult, at least in terms of objective indicators. We can ask whether there has been better access with patient input (obviously, access should not be worse). As importantly, does patient participation lead to development of access criteria that are appropriate, including specification of optimal use, right patients, realistic start-stop criteria, and realistic outcome measures?

Subjectively, one can ask whether the opportunity for input has led to better acceptance by patients and patient groups of allocation decisions, in particular, negative, restrictive, or delayed decisions. Indeed, patient protests and public demonstrations of HTA recommendations in countries like Scotland, England/Wales, and Canada, which ostensibly offer the highest degree of integration across the HTA spectrum, belie that conclusion.

The participation of patients (and advocates) can also be examined from the perspective of each stakeholder's goals for patient involvement. The following

briefly summarizes the overarching goal, the benefits, and the potential risks for each stakeholder. The sponsors of HTA (usually the payers) have both a political goal of optimizing the approval of their constituents (voters or insurance plan sponsors) and a business goal of assuring sustainability or profitability of their medicines programs. What they can gain from patient involvement is support (or lack of acrimony) for allocation decisions as well as adherence to usage guidelines, which (if accurate) would ultimately achieve health outcomes and optimal use of resources. Conversely, patient participation could lead to unrealistic expectations as well as disagreement with HTA decisions.

HTA bodies are themselves stakeholders with an acknowledged political goal of delivering justifiable recommendations that also meet the budgetary concerns of their sponsors. From the perspective of the technocrats, the goal is to ensure these recommendations are credible and defensible. To the degree that patient-experienced evidence does add to the scientific evidence and enhances the overall data interpretation and synthesis, the quality of the assessment is improved. The potential risks include bias or dilution of the data or process as well as time delays and resource demands.

The providers of technology, usually shareholder companies but also researchers, are often seeking recommendations that would make the technology available to as many users as (appropriately) possible to generate highest return on investment and/or increased research investment. It is anticipated that patient input would generate rationale for use, articulate preference for new technologies, and apply pressure for broader access. The challenges are potential time delays and the resources required as well as perceived conflict of interest if there is too much interaction.

The users of the technology include a broad group of stakeholders from patients and caregivers to patient organizations and healthcare providers. The common goal is to have access to the "best" technology (matched individual desired outcomes), which is also affordable and sustainable. To the degree that patient involvement results in usage guidelines that are based on (realistic, informed) outcomes and values important to patients, patients and carers will accept and adhere to treatment options. The challenges for the patient stakeholder are access to information, time, and resources to educate "ordinary patients" in order to provide meaningful input and influence post-HTA recommendation.

How could stakeholder goals be shaping patient involvement in each of the countries presented here? In countries where the sponsor at the political level has a legislated or institutionalized mandate for patient (public) involvement in health policy, namely, England/Wales, Germany, and Scotland, the HTA bodies have evolved highly structured and clearly defined mechanisms for patient consultation and input. Brazil and Taiwan with similar legislated mandates but at an earlier stage of HTA development have also provided structured online tools for patient input, albeit in simpler forms. In all of these settings, individuals have an opportunity to contribute (either through designated patient networks or by direct outreach to individuals). In these jurisdictions, there are efforts sponsored by HTA bodies to educate about HTA and the mechanisms for contribution (although efforts may be just starting in Taiwan). In the more developed HTA bodies (NICE, SMC), there have also been efforts to work with the patient organizations to improve communications, design

input tools, and promote opportunities for comment on summaries of the patient submissions or recommendations. Accountability to the patient community (through patient organizations or networks) appears to be a cornerstone of the legislated or rights-based process. Thus, we anticipate that HTA bodies in Brazil and Taiwan will follow through on at least some of these emerging best practices.

In some jurisdictions, HTA bodies only really consider traditional clinical and cost-effectiveness evidence from literature reviews or submissions and may be "arms-length" from the sponsoring entity (government or healthcare provider), such as the case with IQWiG in Germany, CADTH in Canada, TLV in Sweden, PBAC in Australia, and AGENAS in Italy (Chaps. 19, 21, 26, and 28). In these settings, patient involvement may be limited, influenced by the perception that patient input is of lower quality (grounded in individual experience and potentially biased) and that it is difficult to integrate (often qualitative) patient input with scientific evidence.

Another confounding factor is the belief that the goals of the sponsors/payers (and their HTA institutions) are in direct conflict with those of the users (primarily patients but also healthcare providers). Thus, there is a tension created between patient-user goals of optimal individualized access and the plan sponsors' goal of efficient allocation based on modal needs (not outliers).

As noted previously, in these settings, patient input may be collected by the HTA bodies through secondary sources or from "expert" patient entities to meet quality standards and to reduce disease-specific patient bias (Italy, Sweden, Denmark). This is the case in Sweden: where patient involvement is organized by TLV and mostly consultative, with patient groups serving as "references" to provide context information and input on prioritization, and serving on governance board (but not appraisal committees). In many cases, patients raise skepticism about perceived tokenistic involvement with limited transparency and impact on HTA recommendation.

In some countries, patient advocates are trained to produce better quality (less biased) input. In Italy, the Summer School for Civic Leaders in HTA was organized by citizens' organizations (Cittadinanzattiva/Active Citizenship) and delivered with the support of staff and researchers from the HTA body.

Finally, the perceptions of an overly close alignment between the health technology developers and patient interests may be used to contain or minimize patient involvement. To that end, patient organizations may be required to declare potential conflicts of interest based on contributions from pharmaceutical companies (Canada, England/Wales, Scotland). In other jurisdictions, the role of the health technology developer to influence HTA is also limited (Brazil, Taiwan, Sweden).

32.4 Changes in HTA and Future Patient Involvement

Despite the burgeoning evidence of increased involvement, it is important to ask whether patient involvement is fundamentally changing how HTA arrives at recommendations. Otherwise, what is the point? In parallel processes, we can point to the

influence of patient input on technology innovation (patient-focused medicine discovery), clinical trial design (e.g., no placebo, crossover designs, and shorter trials), patient outcome measures (use of patient-reported outcomes including quality of life), regulatory assessment of benefits and risks (respecting patient tolerance for risks as trade-offs to addressing unmet needs), and even post-market monitoring, for example, through enrolment in patient registries (Hoos et al. 2015). Nevertheless, has patient-submitted evidence (quantitative and qualitative) and patient participation in HTA committees changed the assessment of clinical or economic evidence, the dialogue around the evidence, and the deliberation to arrive at a decision?

As noted by Hailey (Sect. 31.5.3), "But beyond this basic level there are issues of to what extent patient involvement has influenced the preparation of HTAs and the decisions that they inform." We recognize it is difficult to identify let alone measure indicators of impact, namely, in terms of decision outcomes. It is not possible to conduct a controlled experiment, and even historical control does not provide much help, since many other factors have also changed in terms of the technologies and the evidence, thus the limitations of quantitative scientific inquiry. While we could do a qualitative reading of process and recommendations pre- and post-patient involvement or compare between institutions with and without patient involvement, few institutions provide the requiste level of detail, especially those without a patient component, hence the lack of qualitative data for analysis.

One particular area where patient involvement might be expected to have an important influence is the assessment of technologies with high uncertainty attached to the available evidence such as breakthrough therapies, potential cures including gene therapy, medicines for rare diseases or niched population and often with high per-patient costs, and lack of standardized comparators. There is little evidence that HTA has been amenable to change that would better assess the "place in therapy" of these technologies and thus their true value.

NICE in England and Wales has provided a separate pathway for "highly specialized technologies" for very small patient populations, which does not apply traditional HTA methodologies of assessment or appraisal (NICE 2016). Scotland has established a Patient and Clinician Engagement process to evaluate medicines for end-of-life and rare conditions (SMC 2014). Australia has created the Life Saving Drugs Program (Australian Government Department of Health 2016) to provide access to "expensive and life saving drugs for serious and rare medical conditions." There have been similar schemes in other jurisdictions, many at regional and hospital levels but none contributing to a fundamental change in how HTA is applied to innovative technologies.

It has been noted that "[u]ntil recently, HTA has relied on RCTs,[1] systematic reviews, meta-analyses, and cost-effectiveness analyses, all conducted before market entry" (Lewis et al. 2015 [4116]). And while managed entry arrangements, including risk sharing, pay for performance, and coverage with evidence development, have been used in Europe, North America, and Australia, especially to provide "early access" to medicines for illness that are serious or life threatening, these have not been systematically or consistently employed (Stafinski et al. 2010). Thus,

[1] Randomized controlled trials

even as patient involvement is increasing, there is little evidence that HTA processes are themselves evolving not only to better accommodate patient-based evidence and participation but also to meet the assessment needs of new technologies. Even the most cursory application of "change models" suggests no shift is taking place. According to Lewin's three-stage model (Schein 1996), there would need to be some indication of "unfreeze" from current methods, "change" toward new methods, and subsequent "refreeze." Similarly, Adkar's stages of change start with an "awareness of the need to change" followed by the "desire to change" as preludes to gaining the knowledge, expertise, and reinforcement for change (Prosci 2016). No actions indicative of this change process are apparent with the HTA institutions although innovative programs like the IMI's ADAPTSMART (2016) are being piloted in Europe.

Similarly, in low- and middle-income countries where universal health coverage is newly emerging, a legitimate concern is whether HTA will lead to better allocation of limited healthcare resources, as has been suggested by the World Health Organization (2016) among others. Or can an "enlightened" HTA process also allow for the introduction of new technologies, as desired by the patient groups? Moreover, what will be the role of patient involvement in establishing the goals of healthcare and health resource allocation? Finally, if patient input contributes to a quintessential HTA process based primarily on RCTs, quantitative QOL scales, and cost-effectiveness and cost-utility measures, it may result in outcomes that will justify rationing of healthcare without real consideration of patient goals and patient values, and this may not lead to either better outcomes or to better acceptance.

References

ADAPTSMART. Accelerated development of appropriate patient therapies. 2016. http://adaptsmart.eu/the-european-medicines-agency-on-adaptive-pathways/. Accessed 20 Oct 2016.

Australian Government Department of Health. Life saving drugs program criteria and conditions. 2016. http://www.health.gov.au/internet/main/publishing.nsf/Content/lsdp-criteria. Accessed 20 Oct 2016.

Hoos A, Anderson J, Boutin M, Dewulf L, Geissler J, et al. Partnering with patients in the development and lifecycle of medicines: a call for action. Ther Innov Regul Sci. 2015;49:929–9.

Lewis JRR, Kerridge I, Lipworth W. Coverage with evidence development and managed entry in the funding of personalized medicine: practical and ethical challenges for oncology. J Clin Oncol. 2015;33:4112–7.

NICE. NICE highly specialised technologies guidance. 2016. https://www.nice.org.uk/about/what-we-do/our-programmes/nice-guidance/nice-highly-specialised-technologies-guidance. Accessed 20 Oct 2016.

Prosci. Adkar change management model overview. 2016. https://www.prosci.com/adkar/adkar-model. Accessed 20 Oct 2016.

Schein EH. Kurt Lewin's change theory in the field and in the classroom: notes toward a model of managed learning. Syst Pract. 1996;9:27–47.

Scottish Medicines Consortium. PACE (Patient and Clinician Engagement) overview document: process for end of life and very rare conditions (orphan and ultra-orphan medicines). 2014. https://www.scottishmedicines.org.uk/files/PACE/PACE_Overview_Document_V2.pdf. Accessed 22 Nov 2016.

Stafinski T, McCabe CJ, Menon D. Funding the unfundable: mechanisms for managing uncertainty in decisions on the introduction of new and innovative technologies into healthcare systems. PharmacoEconomics. 2010;28:113–42.

WHO. Using health technology assessment for universal health coverage and reimbursement systems. 2–3 Nov 2015, Geneva. 2016. http://www.who.int/health-technology-assessment/HTA_November_meeting_report_Final.pdf. Accessed 20 Oct 2016.

Chapter 33
Patient Involvement in Medicine Development and Assessment

David L. Grainger

33.1 Introduction

Experts in regulatory and HTA agencies are urging the pharmaceutical industry to make more effort to include patients' perspectives in the medicine development process to further improve the quality and relevance of evidence of effectiveness and value. Indeed, much of the product development activity within companies is geared around meeting goals for regulatory approvals and market access. Therefore, the pharmaceutical industry has an interest in responding to changes in how these agencies consider evidence and make decisions. Many companies make considerable effort to involve patients during the development process as an essential part of improving the overall efficiency of that process. The pharmaceutical industry is benefiting from insights that improve the efficiency of medicine development, while ensuring patient organisations are able to become involved in regulatory and HTA processes. International and national guidelines provide governance to the process and ensure this involvement will occur transparently, without influence and without any overt promotion of specific products (European Federation of Pharmaceutical Industry Associations 2011; Perfetto et al. 2015). This chapter will review the evolution and value of patient involvement in medicine development and assessment processes, discuss legal constraints on communication between the pharmaceutical industry and patients and the importance of patient education and training.

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33.2 Historical Perspective

Patients as individuals have been growing more empowered and involved in the health system, particularly due to the increased access to health-related information via the Internet (Dewulf 2015). Patient organisations have also emerged as sources of support and education for individual patients as well as powerful advocates and lobbyists for the interests of their constituents (Chap. 35). While this initially focused on overall access to quality care, the issue of timely and affordable access to new treatments has emerged as one of the main interests of patient groups. The pharmaceutical industry shares this objective of timely and affordable access.

In most jurisdictions there is concern that involvement between the pharmaceutical industry ('the industry') and patients may result in undue and inappropriate influence on their decisions. Other than in the United States and New Zealand, direct promotion to patients is illegal. While reactive communication (responding to patient calls for specific information or conducting clinical trials) is permitted, it is heavily regulated to ensure patients are protected from inappropriate promotion.

This evolution of the voice of patients and their relationship with the pharmaceutical industry has been the subject of attention in the past decade. As HTA grew as the 'fourth hurdle' on the way towards affordable access, the industry was accused of attempting to capture the policy agenda of patient groups. While a study in the United Kingdom failed to confirm this was the case, it did conclude that 'the shallow approach to transparency adopted by the majority of companies and groups strengthens critiques of undue influence' (Jones 2008 [941]).

Fortunately, there has been a maturing of the relationships. Many industry codes of conduct now require acknowledgement of financial relationship between companies and patient groups (Francer et al. 2014). Multiple stakeholders call for frameworks for the involvement of patients in medicine research and development and assessment of value. Barriers to increased patient involvement include a lack of resources and expertise at patient organisations given the wide range of policy issues where their contribution is sought. There is therefore a need for education and training of patient advocates, and in the absence of support for this from health systems, industry is often called upon to fund such programmes. This may be an appropriate role provided transparent, independent third-party experts are used, and specific medicines or technologies are not the focus. For example, the European Patients Academy on Therapeutic Innovation (EUPATI) was established in 2012 to deliver training to patient representatives across Europe. This was a patient-led initiative supported by a multi-stakeholder consortium of patients, academia, industry and not-for-profit organisations (Pushparajah et al. 2015).

33.3 Patient Involvement in Evidence Development

The pharmaceutical industry needs to better manage the time and cost of clinical trials and seeks to improve patient recruitment, adherence to protocols and reducing drop out.

Many companies now recognise that developing compelling evidence of gains in patient-relevant outcomes requires early involvement of patients. Bringing patient insights to the product development process helps focus clinical trial design on the most appropriate endpoints (clinical and patient-reported outcomes) and eases trial participation. Industry has a social contract with patients in regard to participation in clinical trials and must ensure participation is an acceptable burden. Lengthy study visits, extensive surveys and collection of seemingly meaningless data all impair trial participation and completion. The pharmaceutical industry has a vested interest in efficient and timely completion of such trials and now values increased patient involvement. In addition, industry needs to grasp the opportunity to improve some specific trial contexts such as paediatric indications, where parental input would lead to improved protocol design. Collaboration between patients with experience of the condition under study and clinical trial teams means the patient perspective is more likely to be integrated into protocol development. Trial reports are more likely to include outcome measures that show where the new technology adds value (Hoos et al. 2015). This collaboration is vital for patient needs other than routinely measured clinical endpoints (e.g. fatigue or difficulty in taking medication). Involvement of patients may take multiple forms including surveys, expert panels and online engagement.

More recently, some patient groups have recognised they can add more value to the overall process by contributing during medicine development than they can at the HTA stage (Low 2015). An example of this is the United Kingdom's Melanoma Foundation, which has developed sophisticated capability to provide patient input to all phases of clinical trials. They established a clinical trial network involving 35 hospitals across the United Kingdom, providing opportunities for patients to contribute to the development of studies via this network. The pharmaceutical industry is partnering with such organisations, recognising it as an efficient and effective way of improving trial design.

Regulators have also developed mechanisms to involve patients in their processes to inform the advice they give on the design of clinical trials and in their assessment of efficacy and safety. Examples can be seen in the Patient Focused Drug Development initiative in the United States (US FFDA 2016a) and EMAs work with patients (EMA 2016).

Overall, industry can learn more from various public sector efforts to include patients in the design and implementation of clinical trials, guideline development and even grant review processes. Some of these efforts are critical of the 'one off', single event nature of patient involvement, advocating instead for more continuous involvement throughout a trial. The pharmaceutical industry efforts could benefit

from consideration of these recommendations, as the industry approach is currently more 'one off', for example a meeting to review a protocol design. The FIRST model reported by rheumatology researchers is an example of the more considered structural involvement from which industry could benefit (Hewlett et al. 2006).

33.4 Demonstration of Value

It is increasingly recognised that patients can contribute to the HTA process in two areas: the experience of living with the particular condition that the new technology is intended to treat and preferences for general therapeutic approaches or specific attributes of the technology (Facey et al. 2010). This is reflected in both building patient insights into clinical trials and encouraging patients and advocates to bring these insights to HTA processes.

Patients' perspectives help understand how important (or not) a particular change in a clinical trial endpoint may be for patients outside of the trial setting. The change in the endpoint may be statistically significant, but how important is that to patients? Does it really matter? An often-quoted example is that of psoriasis. Regulatory standards refer to scores of the reduction in affected skin areas overall. However, patient input highlighted that what matters is the improvement of areas of the skin most often seen, such as the face and hands (Staley and Doherty 2016). This has led to a change in how industry conducts trials in this condition, with increased use of multiple instruments to assess quality of life (QOL) as well as patients' perspectives on improvements in skin appearance.

33.5 Challenges in Patient Valuation of Specific Benefits of a Medicine

Patient input in assessing value is especially important when the technology may have attributes that improve overall patient outcomes, e.g. increased comfort, functioning or easier use that improves long-term adherence. The pharmaceutical industry develops medicines and devices to improve these areas, recognising that in chronic diseases this may mean better long-term health outcomes. These attributes have a value that should be assessed but may require a different perspective from the outcomes that generally arise in the confirmatory clinical trials. This value may be assessed using economic evaluation techniques such as discrete choice experiments (Chap. 10).

The pharmaceutical industry is increasingly considering patient preferences. For example, the IMI proposal for a new project called PREFER to develop recommendations on how the results of patient preference studies could be incorporated in applications for evaluation by regulators, HTA bodies and payers (European Cancer Patient Coalition 2016). At this point, there are 17 companies participating in this collaborative project. Another example is funding academic groups to undertake

discrete choice experiments to elicit preferences for specific HTA submissions. The industry sees a need for guidelines for dossier development in HTA systems to formally acknowledge such analyses. In addition, industry researches patient insights into conditions and treatments using a range of qualitative techniques and social media. Much of this is unpublished and could be streamlined for incorporation into HTA submissions.

A further consideration is the assessment of changes in health-related QOL. Ideally, these are measured during the main clinical trials, using a standardised QOL questionnaire. The pharmaceutical industry has been criticised for the lack of inclusion of QOL instruments in trials. These are now commonplace as a result of HTA body feedback, although it undoubtedly adds to trial costs. However, it is still possible the questionnaire lacked sensitivity to detect effects related to a particular product attribute. Other forms of patient input to the overall HTA process then become even more important. The pharmaceutical industry and patient advocates sometimes challenge the status quo for measurement of QOL in clinical trials, recognising that many instruments are burdensome and difficult to apply to subsequent analyses (e.g. utility assessment).

In addition to QOL assessments and elicitation of patient preferences, PRO continue to receive attention. While the industry now uses improved instruments to better capture PROs, it struggles with the trade-off between complexity and burden in trials versus the value obtained. This complexity can be seen in the *FDA Roadmap to Patient-Focused Outcome Measurement in Clinical Trials* (US FDA 2016b).

33.6 Pharmaceutical Industry Perspective on Special Cases: Oncology and Rare Diseases

Some types of medicines and some disease areas create special challenges for HTA (Rosenberg-Yunger et al. 2012). These challenges are especially apparent in oncology. US pharmaceutical industry pipelines today contain approximately 800 compounds for oncology, suggesting the role of patients in this area deserves special attention (PhRMA 2016). The pharmaceutical industry also has to balance smaller trial size and low patient numbers with the need to develop convincing evidence of value. Surrogate endpoints such as progression-free survival are increasingly common outputs of oncology trials, while traditional clinical endpoints and QOL scales may not capture all of the outcomes important to patients and caregivers.

Rare diseases present similar challenges. Increasingly, HTA agencies are recognising there may be additional dimensions of value associated with patient experiences of rare diseases and associated treatments (Hughes et al. 2016). This is exacerbated by limited evidence of clinical effectiveness. Some agencies have considered multi-criteria decision analyses to help manage this (Sussex et al. 2013), and others utilise strong deliberative processes, considering a wider range of evidence and information as a more practical alternative (NICE 2016). The pharmaceutical industry welcomes these developments as recognition of the challenge to develop a robust evidence base when trials are small. While these developments are encouraging,

they require processes for input from patient advocates and patients. This in turn requires open and appropriate communication between product developers and patient advocates.

33.7 How Pharmaceutical Industry Engagement Reflects Differences Between Countries

It is well known that HTA processes differ considerably between jurisdictions (Muhlbacher 2015). In some, the HTA body controls all aspects of the process from application for reimbursement through to pricing negotiation and post-reimbursement reviews. In other cases, the HTA process may be more separate. It completes assessments of new technologies that are then taken up by a separate agency for pricing and reimbursement decisions. This influences how stakeholders become involved with these processes and on how industry views this engagement.

In the former, the manufacturer triggers the process and is likely to communicate to patient organisations that the technology has been submitted. In the latter, mechanisms are needed for patients to become aware of the selection of technologies for review. This needs clear processes on patient involvement and the industry has an interest in this awareness process. For example, if it involves publication of committee agendas in advance, the industry will want to balance commercial competitive concerns alongside providing sufficient lead time for patient organisations to respond.

Some HTA systems allow patient involvement via several mechanisms. Each has relevance to the interaction between patients and industry. One approach is the presence of one or more patient advocates on appraisal committees. In this instance, the advocate may interact with multiple patient organisations and receive submissions from individual patients and organisations. While industry applauds this, published data may be limited at the time of the first assessment. This may require increased dialogue between the patient representative on the committee and the manufacturer. Likewise, if the patient representative reviews submissions from individual patients and organisations, any support received from the manufacturer needs to be transparent and appropriate.

Another option is submissions from patients and organisations via a web-based template. Again, associations with the sponsor company need to be disclosed. It is also helpful for patients submitting via such templates to be able to review information on the new technology, if local regulations and codes of conduct permit this.

33.8 Barriers to Effective Communication Between Manufacturers and Patients

As described in 34.2, in most countries patients have been ‘protected’ from communication directly with the pharmaceutical industry. This is to prevent inappropriate promotion which might bypass the role of the medical practitioner as prescriber.

However, an unintended consequence is that it is difficult for companies to provide information on products that might be useful to patient advocacy organisations in making submissions to or otherwise becoming involved with the local HTA process.

In response to this, some agencies act as a trusted intermediary, receiving information from the pharmaceutical company in a set format and making it available to patients for these purposes of involvement. The Scottish Medicines Consortium has had such a mechanism in place for many years (SMC Guidance for Submissions 2016), and it is also emerging in evolving HTA processes such as Taiwan (Chap. 29). Several codes of conduct address this issue (Francer et al. 2014). Some have been developed by umbrella patient advocacy organisations such as the International Alliance of Patient Organisations (IAPO), guiding member groups on how best to engage with companies. Likewise, national and regional industry association codes of conduct and 'user guides' can help member companies engage with patient groups. However, there is a trend towards HTA being applied earlier in the process of medicine review and approval. For example, Australia now allows parallel submission to both the national regulatory agency (the Therapeutic Goods Administration) and the national HTA organisation for medicines (the Pharmaceutical Benefits Advisory Committee). Patient advocates may be in a position to comment on a medicine that has not yet received its final regulatory approval, and in such circumstances companies are legally prevented from providing any information on that medicine to patients.

33.9 Capability Development, Training and Education

In addition to the communication of appropriate information on a new technology under HTA review, there is also a role for the pharmaceutical industry to support capability development, education and training. Even in HTA systems that recognise the importance of patient involvement, there are few public resources to support that involvement. Therefore, the pharmaceutical industry has filled that gap in some jurisdictions. Provided it is at arm's length and is transparent, this serves the need and enables patient involvement where otherwise it may not occur.

There are examples of successful programmes supported by the pharmaceutical industry and delivered to patient advocates via an appropriate third party. One is the annual programme delivered by the London School of Economics, supported financially by several companies and attended by patient advocates from across Europe (Training and Capacity Building, European Federation of Neurological Associations 2016). It provides education on the basics of HTA and equips advocates for involvement. Companies have also worked directly with experts in this area to develop online or in-person resources designed to introduce HTA concepts and help patient advocates engage with their local agency. The eMEET programme is an example of this (Medicine Evaluation Educational Training 2016). This virtual resource is freely available on the HTAi website and has been used by some patient organisations to

educate their members, for example in Taiwan. The pharmaceutical industry also provided an unrestricted research grant to support the development of the *HTAi Values and Quality Standards for Patient Involvement in HTA* (Chap. 1).

33.10 Conclusion

The increasing involvement of patients and patient advocates in regulatory and HTA processes is a positive trend to be welcomed by the pharmaceutical industry. More work is needed to optimise the value of this input. However, the pharmaceutical industry recognises the importance of capabilities within advocacy organisations to enable patients' perspectives to be considered. More support for education and training will reduce the 'noise' of demands for access to a particular technology and replace it with information that is truly useful to an appraisal committee. However, this requires clarity of the role of the pharmaceutical industry as one of the sources of information to help patients and advocates contribute to the HTA process. Due to legislative and codes of conduct limitations, the pharmaceutical industry itself needs to be mindful about information it can convey to patients about a new technology and when it can convey it. Likewise, it is critical that interaction between the pharmaceutical industry and patients in no way diminishes the value and credibility of that patient input. Finally, there is much to be gained if the pharmaceutical industry expands its efforts to involve patients in the design of clinical trials, recognising it has long-term benefits for reducing trial costs, improving overall efficiency and helping to ensure the relevance of the data being generated.

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References

Dewulf L. Patient engagement by pharma—why and how? A framework for compliant patient engagement. Ther Innov Regul Sci. 2015;49:9–16.

EMA. Patients and consumers. http://www.ema.europa.eu/ema/index.jsp?curl=pages/partners_and_networks/general/general_content_000317.jsp&mid=WC0b01ac058003500c (2016). Accessed 30 Mar 2016.

European Cancer Patient Coalition. PREFER project: Giving patients a voice in drug development (2016). http://www.ecpc.org/pressroom/news/427-prefer-project-giving-patients-a-voice-in-drug-development. Accessed 28 Oct 2016.

European Federation of Neurological Associations. Training and capacity building. http://efna.net/training/ (2016). Accessed 28 Oct 2016.

European Federation of Pharmaceutical Industry Associations. Code of practice on relationships between the pharmaceutical industry and patient organisations. 2011. http://transparency.efpia.eu/uploads/Modules/Documents/code_po2011.pdf. Accessed 3 Mar 2016.

Facey K, Boivin A, Gracia J, et al. Patients' perspectives in health technology assessment: a route to robust evidence and fair deliberation. Int J Technol Assess Health Care. 2010;26:334–407.

Francer J, Izquierdo JZ, Music T, et al. Ethical pharmaceutical promotion and communications worldwide: codes and regulation. Philos Ethics Humanit Med. 2014;9:7.
Hewlett S, De Wit M, Richards P, et al. Patients and professionals as research partners: challenges, practicalities, and benefits. Arthritis Rheum. 2006;55:676–80.
Hoos A, Anderson J, Boutin M, et al. Partnering with patients in the development and lifecycle of medicines: a call for action. Ther Innov Regul Sci. 2015;49:929–39.
Hughes E, Morgan R, Burgess A. Equitable access to medicines for rare disease patients in Wales. 2016. http://www.rare-diseases.eu/wp-content/uploads/2013/09/45.-%E2%80%98Equitable-Access-to-Medicines-for-Rare-Disease-Patients-in-Wales%E2%80%99.pdf. Accessed 28 Oct 2016.
Jones K. In whose interest? Relationships between health consumer groups and the pharmaceutical industry in the UK. Sociol Health Illn. 2008;6:929–43.
Low E. Potential for patients and patient-driven organizations to improve evidence for health technology assessment. Int. J Tech Assess Health Care. 2015;31:226–7.
Medicine Evaluation Educational Training. The role of patient advocacy. http://www.meetforpatients.com/ (2016). Accessed 28 Oct 2016.
Muhlbacher A. Patient-centric HTA: different strokes for different folks. Expert Rev Pharmacoecon Outcomes Res. 2015;15:591–7.
NICE. Highly specialised technologies guidance (2016). https://www.nice.org.uk/about/what-we-do/our-programmes/nice-guidance/nice-highly-specialised-technologies-guidance. Accessed 28 Oct 2016.
Perfetto EM, Burke E, Oehrlein EM, Epstein RS. Patient-focused drug development: a new direction for collaboration. Med Care. 2015;1:9–17.
PhRMA. Medicines in development. (2016). http://www.phrma.org/report/list-of-2015-medicines-in-development-for-cancer. Accessed 28 Oct 2016.
Pushparajah DS, Geissler J, Westergaard N. EUPATI: collaboration between patients, academia and industry to champion the informed patient in the research and development of medicines. J Med Dev Sci. 2015;1:74–80.
Rosenberg-Yunger ZRS, Thorsteindottir H, Daar AS, Martin DK. Stakeholder involvement in expensive drug recommendation decisions: an international perspective. Health Policy. 2012;105:226–35.
Scottish Medicines Consortium Guidance for Submissions 2016 https://www.scottishmedicines.org.uk/Submission_Process/Submission_guidance_and_forms/Templates-Guidance-for-Submissions/Templates-Guidance-for-Submission. Accessed Oct 2016.
Staley K, Doherty C. It's not evidence, it's insight: bringing patients' perspectives into health technology appraisal at NICE. Res Involvement Engagement. 2016;2:4. doi:10.1186/s40900-016-0018-y.
Sussex J, Rollet P, Garau M, et al. A pilot study of multi-criteria decision analysis for valuing orphan medicines. Value Health. 2013;16:1163–9.
US FDA. Roadmap to patient-focused outcome measurement in clinical trials. 2016b. http://www.fda.gov/Drugs/DevelopmentApprovalProcess/DrugDevelopmentToolsQualificationProgram/ucm370177.htm. Accessed 29 Nov 2016.
US FFDA. FDA patient focused drug development initiative. 2016a. http://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm347317.htm. Accessed 29 Sept 2016.

Chapter 34
Medical Technologies: Involving Patients in Development and Assessment

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34.1 Introduction

Medical technologies (med-tech) is the term used for health technologies such as medical devices, in vitro diagnostics, medical imaging, health information and communication technologies. Their value is largely determined by their ability to address the needs of patients and care-givers. To understand and leverage that value fully, the context in which med-tech is used needs to be accounted for in its development and assessment. This includes individual patient characteristics and needs, the way healthcare systems are organized and the experience of the users. Involving patients from the outset in the design and development of med-tech allows their specific needs to be considered, so safer and better-suited medical technology can be developed and its value determined. Patients can recount any usability problems and facilitate functionality improvements of med-tech. Patient involvement in HTA

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can help to tailor the assessment, allowing wider aspects of the technology's value (clinical, social and economic) to be considered. This chapter sets out the need for patient involvement throughout the process, from design and development of med-tech to HTA. It explains the challenges and provides examples of how patients are involved in the development and assessment of different medical technologies.

34.2 Patient Involvement in the Development of Med-Tech

Patients are generally involved early in the development of med-tech products and this can improve their satisfaction when medical technologies are made available for use (Coulter 2008, Wressle and Samuelsson 2004). Patients involved in development can feel empowered and in control of their care (Money et al. 2011). This can lead to better compliance and improvement in self-management of chronic conditions such as diabetes, where diagnostic technologies are used at home to guide treatment (Maran et al. 2014) which in turn can translate into better health outcomes (Sax et al. 2007). A reduction in device development time may also be seen, avoiding costly design changes and product recalls (De Vito Dabbs et al. 2009). Patients can provide different input according to the type of med-tech in question. Their involvement, together with healthcare professionals', will shape what the final med-tech product will be like. For example, in the case of implantable devices, patient input and evidence is highly relevant to shape their functionality (e.g. how it can be implanted either through minimally invasive or open surgery) to minimize possible negative impacts on daily life and maintain a positive body image.

As shown in Table 34.1 and the case study below it, med-tech products are usually developed through an iterative process where newer, improved versions are continuously developed. Patients and care-givers are involved in every step of this process. The end-results are med-tech products that are better suited to the preferences and needs of patients, care-givers and/or healthcare professionals. These 'tailor made' med-tech products provide a wide range of benefits. This wider value med-tech brings translates into specific considerations for HTA on med-tech which will be the focus of Sect. 34.3.

Case Study: Patients Involved Throughout Med-Tech Design

Devices for Dignity (D4D) is one of eight Healthcare Technology Co-operatives in England in receipt of public funding from the National Institute for Health Research. It brings together academics, clinicians, patients and charities, with innovators from med-tech businesses. A focus of D4D is on ensuring that products preserve dignity and promote independence for product users. D4D's process begins by gauging the opinions of a range of patients and experts within the relevant clinical area, to validate that the unmet

need exists, and is of high priority. D4D then seeks to develop a solution with a development team including patient representatives. The team employs iterative processes to deliver a product that is likely to be commercially successful, within the context of the intended use of the device, including development of evidence to support adoption. An example of D4D's approach is shown by the development of a modular cervical orthosis (neck support) for those affected by progressive neuromuscular weakness. In response to patient dissatisfaction with adjustability and the degree of support offered by current devices, D4D has developed a novel alternative. Individual patients and their care-givers have been involved throughout the iterative design and evaluation phases, including through reviewing of grant submissions and trial design, focus groups, design workshops, and concept and prototype reviews. This ensures that the resulting device fits user needs. Through working closely with a relevant charity the project received wide publicity within the Motor Neurone Disease community, and funding from the charity.

Table 34.1 Examples of the process of patient involvement in med-tech development

Stage	Aim	Techniques used
Stage I—initial concept	When a new project is defined: At country level—user needs and values are established and scoping exercises are carried out (with users) Globally—product requirement document is developed	• Focus group • Individual contextual enquiry (interview with patients in their context, doing their daily activities, to gather raw data for later analysis) • Task analysis and usability tests (cognitive walk-through to see whether a new user can easily carry out a specific task related to a new med-tech product) • Questionnaires • Interviews • Social media
Stage II—validation and refinement	Validate and refine the concept	• Focus group • Individual contextual enquiry • Task analysis and usability tests • Questionnaires • Interviews • Delphi techniques
Stage III—design	Iterative device design based around the needs and preferences of users	Defined by regulatory requirement documents, scenarios, design plan.
Stage IV—evaluation	Pre-launch and post-launch ongoing continuous improvement process. Suggestions and questions about products gathered in countries and then reported through regional channels	• Usability tests • Heuristic evaluation (more holistic usability inspection) • Focus group • Questionnaires • Interviews

34.3 Patient Involvement in HTA Processes for Med-Tech

34.3.1 Medical Devices

From an HTA perspective, patient involvement in the assessment of medical devices relates to how well the device addresses patients' needs. Patients can provide information during the scoping phase of an HTA on issues such as the impact of disease on daily life, experiences with current management strategies to ensure the right comparators are selected, and to select appropriate patient outcome measures. Patients can also provide comments on the functionality and ease of use of the device, and how the device is addressing health needs, the impact on daily activities and adverse events. Patients can contribute to information on what socioeconomic impact, time and resource savings or increase are related to the use of the medical device.

From a methodological perspective, medical devices have specific characteristics that make patient knowledge valuable to HTAs. Medical devices frequently undergo product modifications (Marlow 2016) and there is often a 'learning curve', so a 'steady-state', during which the device can be evaluated in an RCT is unlikely (Drummond et al. 2008). The evaluation of medical devices can be seen as an iterative approach, with revisions as more evidence (e.g. from patients) is gathered on effectiveness in real life. RCTs may also not capture all patient-relevant outcomes—failing to reflect the real impact of medical devices on social functioning and patient well-being—and patient-reported outcomes can face limitations as specific validated tools do not exist for all conditions (Nelson et al. 2015). Patients can contribute information about the benefits of one device over another even when their clinical outcome is similar (e.g. insulin pens vs insulin pump, different kinds of dressings or glucose monitors).

In spite of the importance of involving patients in the HTA of medical devices, only a few countries have set up specific processes. In the case of France, opinions from various stakeholders including patients or citizens have to be considered according to the country's internal process guideline *Common Requirements to all HAS Deliverables* (HAS 2014). The assessment committee in charge of medical devices (CNEDIMTS) includes two patient representatives with voting rights among their members. However, the assessments produced by the CNEDIMTS on medical technologies provide a summary of clinical evidence but do not include a section dedicated to patients' testimonials or any type of qualitative evidence. In addition, as committee member votes are secret, it is difficult to reflect on either the process or the impact of the patient members of the committee.

In England NICE also has a formal process for the inclusion of patients' perspectives (see Sect. 23.6) and examples focused on medical technologies are presented in Sect. 34.4. Most other European HTA bodies do not have formal processes for patient involvement.

At the European level, while patients have been involved in EUnetHTA Joint Action 2 as stakeholders, there have been limited opportunities for patient input. Furthermore, in spite of submission of other forms of patient-based evidence collected by manufacturers, the final reports of the joint HTAs (Rapid Effectiveness

Assessments) have not referred at all to this evidence, considering clinical trials only. The Shaping European Early Dialogues (SEED) Project (Chap 35) has included patients in their meetings to provide scientific advice to med-tech developers. However, it was often difficult to include patients with the right expertise.

34.3.2 In Vitro Diagnostics (IVDs)

IVDs are tests performed on samples like blood or tissue. Unlike a therapeutic technology, they do not influence patient outcomes directly but deliver valuable diagnostic information to choose the best course of action with more certainty (Wurcel and Int. VODI Platform 2016). The main challenge for HTA is how to measure the benefit of that information and account for the related contextual factors (e.g. available therapeutic options after the test is applied, specific care pathways, who will use the information). For example, the QALY has limitations when measuring the value of IVDs, as they do not directly affect the quality of life and/or life years of the patient (Rogalewicz and Juřičková 2014). This highlights the specificity and expertise needed to perform HTAs on IVDs, and could explain why experienced HTA bodies in Europe such as NICE and NHS Scotland have set up specific programmes, guidelines and even decision making frameworks for diagnostics (NICE 2011); (NHS Scotland 2011).

HTA on IVDs begins by establishing the healthcare pathway in which the test will be used. Patients can share critical points of their disease, available healthcare options and potential for improvement. Patients can help HTA to measure the benefits of diagnostic information by providing feedback on the value of a test from a patient perspective. This might include how 'usable' self-administered tests are (e.g. blood glucose or coagulation monitoring), and what patients think is most likely to be gained from diagnostic information. Such gains include: more accurate diagnosis, faster access to test results, better experiences waiting for test results, value of knowing or ability to make choices on health status or prognosis, ability to plan for reproduction/work/retirement with access to diagnostic information, to have a name for their condition, reassurance of non-disease, less intrusive, painful or uncomfortable test, and less impediment of everyday activities with self-testing (Wurcel and Int. VODI Platform 2016, HTAi 2014, 2017).

34.4 Practical Examples of Patient Involvement in HTA for Med-Tech

The example below shows how patients have been involved in the assessment of medical devices and IVDs by NICE.

Example of Patient Involvement in HTA for Medical Technology: NICE

1. Atrial fibrillation and heart valve disease: self-monitoring coagulation status using point-of-care coagulometers

The development of NICE's diagnostics guidance involved two patients as members of the advisory committee. They provided information on the benefits of self-monitoring on psychological wellbeing in having a sense of control over the condition, reducing the need to attend clinics or hospital, allowing patients to travel, visit and care for other family members. Issues with variability of access to self-testing strips in the NHS, that restricted patient's freedom in terms of moving house (as the testing strips may not be available in their new location) were also highlighted. These contributions led the committee to conclude that the cost-effectiveness analyses presented as part of the evidence did not fully capture the benefits for patients (NICE 2014a).

2. Children with scoliosis: the MAGEC system for spinal lengthening

For NICE's evaluation of the MAGEC system a parent of a child who had treatment with the MAGEC system was invited to give testimony to the committee. She was able to highlight the quality of life benefits of the treatment, including that children have a more positive attitude towards the rod lengthening procedure compared with the distress experienced with surgical lengthening, and the need for less time in hospital, less time away from usual activities, less pain and less scarring. She also noted the beneficial peer support that children gained from each other when attending clinics to have the rods lengthened, by being able to interact with each other and reduce their sense of isolation (NICE 2014b). The committee that developed the guidance was able to incorporate this input into its considerations.

3. Benign prostatic hyperplasia: the UroLift system for treating lower urinary tract symptoms

NICE's draft recommendation about the Urolift system was published as part of a consultation. Comments received from patients reinforced the importance of preserving sexual function, in some cases enhancing it, and the impact of that on their quality of life (NICE 2015). These comments added to the evidence base supporting the case for adopting the Urolift system in the UK NHS.

34.5 Barriers to Patient Involvement and Possible Solutions

Patient involvement in med-tech development and assessment may create several challenges for manufacturers and HTA bodies. Table 5.1 outlines barriers to patient participation in HTA. For medical devices and diagnostics, we encounter additional challenges, as well as possible solutions as outlined in Table 34.2.

Table 34.2 Barriers and possible solutions to increase patient involvement in the development and HTA of medical technologies

Barrier	Possible solutions
Heterogeneity and scarcity of HTA processes that include patients' perspectives	• Patient involvement in the development of med-tech should be recognized as key evidence for the assessment of medical technologies. • The fact that the effectiveness of many medical technologies strongly relates to the way the patient interacts with the device should be acknowledged by HTA bodies. • All agencies assessing med-tech should implement active patient involvement processes reflected in specific guidelines and programmes. Patient seats should be guaranteed on each committee that plays a role in assessing medical technologies. • Patient opinions should be sought routinely throughout HTA evaluations, with patients also able to provide input at any additional time.
Organizational change needed to facilitate patient involvement	• HTA bodies need to recognize that they need patient input to comprehensively assess medical technologies, and that steps should be taken and resources available to really listen and reach out to the patient, rather than expecting patients to become experts in making themselves heard or navigating multiple meetings or systems.
How to ensure individual/disease specific patient representation	• As a standard process for all HTAs on medical technologies, HTA bodies should actively search for patients who are willing to provide evidence. • A change is needed on the perception that patients who have a certain condition are biased, they have unique knowledge for HTA on med-tech. • Individual patients can be approached through umbrella organizations or relevant charities. • Wider contact can be improved by using social media and web based platforms.
Difficulties in locating and engaging representative users	• Med-tech developers and HTA assessors should ensure patients and care-givers are given opportunities to provide input from early stages of projects without fear of critical questioning. • Patient input needs to be made as easy as possible, by listening to patients and care-givers on their own terms, and taking active steps to enable patient participation (such as travelling to the patient's home, meeting at their convenience, and providing travel/childcare expenses).
Acceptance of care-givers' or family members' evidence as proxy for patients' evidence	• Clear principles for working with children and/or people with disabilities should be established.
Legal concerns	• A clear legal framework should be in place for interactions between patients, manufacturers and/or HTA bodies.

34.6 Conclusions

Patient involvement is vital and, in general, standard practice during the development of med-tech, allowing new valuable products to be developed with the ability to address patients' unmet needs. Patient involvement in HTA for med-tech is starting to be recognized as a key step to uncover the full value of medical technologies. In particular, the effectiveness assessment of a patient-controlled med-tech device needs to account for the way the patient will use it, and the assessment of the utility of a diagnostic device needs to be guided by benefits patients consider should be gained from diagnostic information. A closer collaboration between patients, HTA bodies and med-tech manufacturers needs to develop, and all resources pulled together to facilitate systematic patient involvement in HTA for med-tech. Patient involvement in the development of med-tech must be recognized and considered as key evidence in future HTAs. It is important that steps be taken to facilitate patient input (Chap. 6) and to generate patient-based evidence (Chap. 4) based on relevant qualitative and quantitative research strategies.

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References

Coulter A, Parsons S, Askham J. Where are the patients in decision making about their own care? Policy brief. World Health Organization. 2008. http://www.who.int/management/general/decisionmaking/WhereArePatientsinDecisionMaking.pdf. Accessed 3 Sept 2016.

De Vito Dabbs A, Myers BA, Mc Curry KR, Dunbar-Jacob J, Hawkins RP, Begey A, Dew MA. User-centered design and interactive health technologies for patients. Comput Inform Nurs. 2009;27(3):–175. http://www.ncbi.nlm.nih.gov/pmc/articles/PMC2818536/

Drummond M, Griffin A, Tarricone R. Economic evaluation for devices and drugs—same or different? Value Health. 2008;12(4):402–4. http://www.sciencedirect.com/science/article/pii/S1098301510607784

HAS. Exigences communesà toutes les productions de la HAS. 2014. http://www.has-sante.fr/portail/upload/docs/application/pdf/2015-02/methodes_has_exigences_communes.pdf Accessed 2 Sept 2016.

HTAi. Patient group submission template–non medicines HTA. 2014. http://www.htai.org/interest-groups/patient-and-citizen-involvement/resources/for-patients-and-patient-groups.html Accessed 1 Sept 2016.

HTAi. Patient group submission template for HTA of diagnostic technologies. 2017 (in press)

Maran A, Tschoepe D, Di Mauro M, Fisher WA, Loeffler K, Vesper I, Bloethner S, Mast O, Weissmann J, Amann-Zalán I, Moritz A, Parkin CG, Kohut T, Cranston I. Use of an integrated strip-free blood glucose monitoring system increases frequency of self-monitoring and improves glycemic control: results from the ExAct study. J Clin Transl Endocrinol. 2014;1(4):161–6. http://www.sciencedirect.com/science/article/pii/S2214623714000325

Money AG, Barnett J, Kuljis J, et al. The role of the user within the medical device design and development process: medical device manufacturers perspectives. BMC. 2011;11:15. http://www.ncbi.nlm.nih.gov/pmc/articles/PMC3058010/

Marlow M. Evidence development and decision making for medical devices. Value & Outcomes Spotlight. 2016. http://www.ispor.org/health-policy_decision-making_medical-devices.pdf Accessed 4 Sept 2016.

Nelson E, Eftimovska E, Lind C, Hager A, Wasson JH, Lindblad S. Patient reported outcomes in practice. BMJ. 2015:350. http://www.bmj.com/content/350/bmj.g7818.long

NHS Scotland. Molecular Pathology Evaluation Panel. Criteria for decision making. 2011. http://www.nsd.scot.nhs.uk/services/specserv/molpath.html. Accessed 21 Aug 2016.

NICE. Diagnostic Assessment Programme Manual. 2011. https://www.nice.org.uk/Media/Default/About/what-we-do/NICE-guidance/NICE-diagnostics-guidance/Diagnostics-assessment-programme-manual.pdf Accessed 1 Sept 2016.

NICE. Diagnostics guidance [DG14] – Atrial fibrillation and heart valve disease: self-monitoring coagulation status using point of care coagulometers (the CoaguChek XS system and the INRatio2 PT/INR monitor). 2014a. http://www.nice.org.uk/guidance/dg14/chapter/6-Considerations. Accessed 1 Sept 2016.

NICE. Medical technology guidance [MTG18], The MAGEC system for spinal lengthening in children with scoliosis. 2014b. http://www.nice.org.uk/guidance/mtg18/chapter/3-Clinical-evidence Section 3.23. Accessed 2 Sept 2016.

NICE. Medical technology guidance [MTG26], UroLift for treating lower urinary tract symptoms of benign prostatic hyperplasia. 2015. https://www.nice.org.uk/guidance/mtg26/chapter/About-this-guidance. Accessed 1 Sept 2016.

Rogalewicz V, Juřičková I. Specificities of medical devices affecting health technology assessment methodology. Proceedings IWBBIO Granada. 2014. http://iwbbio.ugr.es/2014/papers/IWBBIO_2014_paper_130.pdf. Accessed 2 Sept 2016.

Sax H, Allegranzi B, Uçkay I, Larson E, Boyce J, Pittet D. My five moments for hand hygiene: a user-centred design approach to understand, train, monitor and report hand hygiene. J Hosp Infect. 2007;67:9–21.

Wurcel V. on behalf of the International VODI Platform. The value of diagnostic information: uncovering the full value of diagnostics, 2016 (in press).

Wressle E, Samuelsson K. User satisfaction with mobility assistive devices. Scand J Occup Ther. 2004;11:143–50. http://www.tandfonline.com/doi/abs/10.1080/09638280701355777

Chapter 35
Role of Patient Organisations

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35.1 Introduction

For patient organisations, participation in HTA provides an opportunity to contribute meaningfully to decisions surrounding access to health technologies and their potential reimbursement or use (Wong-Rieger 2013). One key benefit of patient participation in HTA is to challenge the adequacy and transparency of HTA processes. Such challenges include the degree to which a specific HTA includes aspects that are important to patients and families living with the condition and how they are considered in the HTA. This chapter describes some of the roles played by patient organisations in HTA, particularly focusing on umbrella patient organisations. It describes the challenges for patient organisations caused by international variations in HTA, skill and resource requirements and attitudes among decision-makers towards patient involvement. It sets out how patient organisations are working to meet these challenges through collaboration,

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capacity building and influencing HTA methodologies and frameworks. Finally, the chapter reflects on ongoing challenges for involvement that patient organisations would describe as meaningful.

35.2 Patient Organisations in Context

The role of patient organisations in the research, regulatory and policy environment is transformative and has evolved considerably in the past 30 years. Patient involvement in medicine approval and access issues was pioneered by HIV/AIDS activists who led a rights-based global movement (Marcus 2011) in the 1980s and 1990s. Inherent to their strategy was the concept of patient empowerment. The current status of patient involvement in HTA finds its foundations in the advocacy work started in the 1990s when the high prices of medicines became a public issue. Some healthcare systems/providers started to request cost-effectiveness studies for new medicines. For example, potent anti-HIV products were authorised in 1996, and at that time EU member states with effective compassionate use schemes had already provided treatments to most patients in greatest need (e.g. 11,000 in France). In contrast, patients living in countries where authorities had requested cost-effectiveness studies were still waiting for the life-saving treatment to become available (EATG 1996). AIDS patient organisations became therefore active interlocutors of the health service discussing the value and cost of such treatment.

The credibility and legitimacy of patient organisations involved in such processes are key. Requirements may include specific funding arrangements (Canadian Organization for Rare Disorders—CORD 2016) and a set of defined criteria including representativeness, transparency and accountability (EMA 2016a). Furthermore, patient involvement in HTA has implications for how patient organisations use their limited resources and prioritise the capacities they need to develop. For example, patient organisations have identified the time-intensive nature of patient involvement and lack of capacity as barriers to patient organisations becoming involved in HTA. Contextually, according to research conducted by the European Patients' Forum (EPF), the involvement of proxies, such as doctors, is not a solution to overcome those barriers (EPF 2013). This research pinpointed (a) the need for education of patients and the wider HTA community and (b) the need for agreed methodologies and frameworks to facilitate patient involvement.

35.2.1 Umbrella Organisations

Global, regional and national patient organisations, umbrella and disease-specific, are highly diverse and complementary in approach and focus. However, all represent and educate patients and are led and driven by the needs of their members. Umbrella patient organisations play a critical role in encouraging collaboration among patient

groups, supporting capacity building and influencing developments in methodologies and policy, and so they can play a crucial role in HTA. Examples of umbrella patient organisations are the International Alliance of Patients' Organizations (IAPO), European Patients' Forum (EPF) and the European Organisation for Rare Diseases (EURORDIS). IAPO is a global alliance representing patients of all nations across disease areas and promoting patient-centred healthcare (IAPO 2016). IAPO began looking specifically at HTA to advocate for and build capacity of its members in the mid-2000s. At the regional level, EPF is an umbrella organisation that brings the collective patients' voice to the EU health and social policy discourse (EPF 2016). EURORDIS advocates for more coordinated decisions on reimbursement for orphan medicinal products. Its 'EurordisCare' (EURORDIS 2009) and 'Price and Availability' surveys (EURORDIS 2016a) demonstrated huge gaps in accessing newly authorised orphan medicine in the EU. EURORDIS encourages its members to establish working relations with HTA bodies in their respective countries and has begun building an international network.

Umbrella patient organisations work across disease areas and geographies with all stakeholders to develop systems and processes that have the potential to maximise real value and benefit to patients and society from new technologies. At the international level, they connect patient organisations across borders to promote good practices in patient involvement and share information relevant to HTA that informs decision-making. In addition, international disease-specific umbrella organisations are increasingly involved in research prioritisation and the design of phase II/III studies in early regulatory dialogue and early scientific advice about clinical trial design as well as new trial methodologies and licencing schemes such as adaptive pathways for medicines and coverage with evidence development. For medical devices, the regulatory requirements for clinical studies vary depending on the form of device, with some devices not requiring any clinical trials before regulatory approval. Furthermore, only a small proportion of medical devices are subject to HTA. As a result, patient groups have not had so much focus on promoting the involvement of patients in the development and assessment of medical devices.

The interplay between national patient organisations and umbrella organisations is crucial. HTA requirements at the national level must be understood in order for umbrella organisations to influence change at early stages in the process such as when research design decisions are taken. For example, many cancer patient organisations are involved in research design, research conduct and regulatory affairs. They are represented at the national, regional and international levels by coalitions (e.g. the European Cancer Patient Coalition) or a single cancer-type international coalitions (e.g. Global Lung Cancer Coalition). At the national level, an example of umbrella patient organisations working with HTA bodies is seen in Scotland. The SMC Public Involvement Network advisory group plays an important role in developing patient training across a range of disease areas (Chap. 27). Looking beyond Europe, Pivik et al. (2004) empirically assessed potential avenues for consumer involvement in HTA in Canada. Since then, CADTH has developed a process, which actively solicits patient organisations' input. The patient organisation input includes direct in-country experience with new medicines and technologies, as well as international patient

experiences gleaned from the national group's relationships with umbrella organisations. In Australia, umbrella organisations such as Rare Cancers Australia play a crucial role in providing patient input and representation to regulatory and HTA bodies (e.g. PBAC) for patients with over 200 rare cancers, many of whom would otherwise not be represented by a formal patient organisation.

35.2.2 International Variation

While collaboration in umbrella organisations enables patient organisations to share skills and resources and strengthen their influence, wide international variations on how HTA is conducted and on how patient involvement is undertaken impose limits. Those from areas with well-developed HTA can share knowledge and experience through umbrella organisations; however, there is also a need to develop tools and strategies which can be adapted to local contexts and culturally specific issues, so that local patient organisations can understand and engage in their local HTA process.

For example, in Latin America, which has had the fastest development of HTA, IAPO has been promoting the HTAi Values and Quality Standards for Patient Involvement in HTA (Chap. 1). It was possible to adapt and use these in all jurisdictions, despite the different interpretations and practices in HTA that have emerged. Their availability in local languages was key, as this triggered conversations and developed common knowledge and collaboration.

Awareness and expertise of HTA are continuing to grow in Latin America, where countries have made some key inroads in the commitment to, and quality of, HTA, including cross-border collaboration as they adapt methods from elsewhere. Patient organisations often highlight that lower cost and increasing access (and the speed of access) to new and innovative treatments and devices have a positive impact for patients. However, they also foresee a risk of lack of contextualised assessment of new technologies and of the socio-ethical impact on the region, as well as financial considerations. This is especially the case for emerging biologic medicines and, for example, the 'abbreviated pathway' in Colombia that could accelerate access to treatments, but has risks in terms of the extent of assessment and completeness of the evaluation of other potential impacts. There is concern that in the presence of regional precedents, broader patient and community needs will not be considered.

Meanwhile, the Asian region has an extremely heterogeneous level of patient involvement in HTA. While Thailand has an advanced HTA system, many other countries are only starting to consider HTA systems, and a patient-centred approach is neither embedded nor common. Therefore, strong patient advocacy can play a key role in contexts where there are no obvious mechanisms for patient participation. In Hong Kong, awareness building and understanding among patient groups has led to collaboration with health professionals to advocate with the government not only for greater patient involvement but also to stimulate discussion of HTA as a tool, within health systems and government.

Low- and middle-income countries are still, by and large, at the basic level of advocating for consistent and genuine patient involvement, transparency of decision-making processes and effective pharmacovigilance and communication, rather than individual or organised intervention in specific HTA recommendations.

35.2.3 Capacity Building

In Europe, between 2005 and 2008 in the context of the High-Level Pharmaceutical Forum, early strategic policy discussions within and between stakeholders[1] led to substantive work to attempt to facilitate meaningful patient involvement in HTA (European Commission 2008).

In 2008, the first guide was produced by Health Equality Europe to help patients and the public understand and get involved in HTA, either directly in disease-specific decisions or to advocate for better consideration of patients' needs. The guide and linked seminars were co-produced with experienced patient representatives, academics and advocacy and umbrella organisations (Health Equality Europe 2008).

Since then, a range of educational initiatives has taken place. In 2010, EURORDIS created a new programme for its summer school for patients' advocates, focusing on HTA (EURORDIS 2016b). This 4-day seminar trains 40–50 advocates in rare diseases every year.[2] From 2011, the European Federation of Neurological Associations (EFNA) worked with the London School of Economics in delivering 3-day courses for patient advocates on HTA. Over 4 years, more than 250 participants took part in these workshops.

Arguably, the most prominent example of capacity building at European level is the European Patient Academy on Therapeutic Innovation (EUPATI 2016a). EUPATI is a patient-led public-private partnership, coordinated by EPF[3] and involving 33 organisations from patient organisations, academia, non-profit organisations and pharmaceutical industry. The partnership is focused on providing educational materials on the therapeutic innovation process in multiple languages. A specific and extensive module has been created that focuses on HTA and patient involve-

[1] Apart from EPF representing patients, involved stakeholder groups were healthcare professionals (Standing Committee of European Doctors (CPME), Pharmaceutical Group of the European Union (PGEU)), payers (Association Internationale de la Mutualité (AIM), European Social Insurance Platform (ESIP)), industry (European Federation of Pharmaceutical Industries and Associations (EFPIA), European Generic Medicines Association (EGA), European Self-Medication Industry (AESGP), European Association for Bioindustries (EuropaBio), European Association of Full-Line Wholesalers (GIRP)).

[2] Part of the training is inspired by a course 'Introduction to Health Technology Assessment' by the Institute of Public Health, University for Health Sciences, Medical Informatics and Technology, Hall in Tirol (Prof. Uwe Siebert).

[3] Other public partners involved in EUPATI are EURORDIS, European Genetic Alliance and European AIDS Treatment Group.

ment. By the end of the project in early 2017, 96 patient representatives throughout Europe have completed an intensive expert level 15-month course, and over 100,000 patients and patient advocates have utilised the EUPATI Toolbox (EUPATI 2016b) developed in more than seven languages, including a major section on HTA processes, which has had input from HTAi, EUnetHTA and NICE.

EUPATI has also developed guidance documents. EUPATI's *Framework for Patient Involvement in HTA* to be published in 2017 specifically covers the interaction between HTA bodies and patients in relation to medicines. It focuses on participation in the HTA process and aims to promote good practice while complementing related work of EUnetHTA, HTAi, patient organisations, academia, HTA bodies and health technology developers.

From 2017, EUPATI, originally funded as a 5-year Innovative Medicines Initiative (IMI) project, will continue as an EPF-led multi-stakeholder programme. It will continue to provide high-quality patient education to ensure systematic and structured patient participation in the life cycle of medicines. Exploratory work is also needed to address the issue of capacity of other players in the regulatory and HTA environment to engage with patients. As the EPF research demonstrated, this remains a significant barrier (EPF 2013).

EPF has an explicit strategy to create more patient leaders across the EU, who can play an effective and critiquing role in HTA policy and practice. To achieve this, in 2010 a multi-stakeholder seminar included 80 patient representatives from around Europe. This seminar demonstrated how umbrella organisations engage with their representatives who can then act themselves and empower other individual patients within their communities. It also confirmed the need for greater collaboration and genuine and systematic patient involvement in HTA and throughout the innovation process to deliver access to more effective new treatments (EPF 2010).

More recently, EPF has developed with MedTech Europe the Patient-MedTech Dialogue which allows ongoing dialogue between patient and medical device industry representatives. In 2015, it focused on HTA to identify barriers and facilitators, good practices and potential solutions to better integrate patients' views in the HTA process for medical devices (MedTech Europe 2015). EPF also sets up an informal working group on HTA to build a vibrant network of patient advocates to feed into HTA policy developments and coordinate actions linked to HTAi, the HTA Network and EUnetHTA.

35.2.4 Influencing HTA Methodologies and Frameworks: the EUnetHTA Example

Collaboration between European HTA bodies in the form of EUnetHTA (Chap. 24) has provided an opportunity for patient organisations to influence patient involvement methodologies and frameworks in Europe. Patient involvement in EUnetHTA has evolved over time. Initially, EUnetHTA was largely academic, developing common methodologies and guidelines for HTA bodies in Europe, with patients consulted on their developments via representation of umbrella organisations on the Stakeholder

Forum. However, as EUnetHTA has moved more towards joint production of HTA information and early dialogues with companies, consultation with umbrella patient organisations and inclusion of individual patients have increased.

A number of core issues have emerged from this work, including:

- Capacity of patient organisations to engage in HTA-related activities
- Points at which patients engage and the differing roles of European and national patient organisations
- Consultation processes
- Transparency
- Need for patient involvement in the drafting of lay person summaries
- Importance of early involvement of patients and early dialogue
- Need for an online patient organisation-led platform allowing communication and exchange of information to identify and support patients
- Need for training and education

Shared learning is key. Other European initiatives or institutions have already developed models of patients' and consumers' participation. For example, EMA implements a successful model of patient involvement including rules for involvement and policies (e.g. financial arrangements, directory of patients' and consumers' organisations, policy on conflicts of interests, reporting, working groups, etc.) (EMA 2016b).

Developments in EUnetHTA are also linked to the EU Directive on *Patients' Rights in Cross Border Healthcare* that led to the establishment of the HTA Network (European Commission 2012), a member state-led network on HTA that provides political and strategic guidance for HTA collaboration in Europe (whereas EUnetHTA provides the scientific and technical work).

The HTA Network is currently defining new approaches for the involvement of patients and consumers, with the creation of its own Stakeholder Forum.

There is increasing emphasis on joint scientific advice, early dialogue and real-world evidence. A successful example in this context is Shaping European Early Dialogues (SEED 2015), a project led by the French HTA body together with 12 others, providing scientific advice to the developers of new technologies. Their aim is to reduce the risk of inadequate data when products are presented for evaluation by national health insurance bodies. The project provided important learning regarding the time investment needed to identify, brief and accompany patient experts and the training and support requirements to empower them.

The IMI project ADAPTSMART (2016) brings together relevant stakeholders, including patients and HTA agencies to explore how to advance the concept of Medicines Adaptive Pathways to Patients (MAPPs), with a focus on evidence generation throughout the entire product life cycle, designing the MAPPs pathway and discussing decision-making, sustainability and ethical and legal implications. This brings new challenges to the patient community. It requires new skill sets to be able to:

- Engage effectively as an equal partner at all stages in the process
- Be part of the early dialogue that will influence trial design, end points and expectations of regulators, HTA experts and payers

- Contribute to the collection of real-world evidence

EPF and EURORDIS are actively involved in this project and will assess how best patients can be supported to navigate this new terrain. Other organisations, such as the European Multiple Sclerosis Platform (2016) through their project EUReMS, have demonstrated the important role of international registries and the significance of epidemiological, clinical and disease management data collection, including crucial quality of life data. With increasing emphasis on collection of real-world evidence, the critical role of such registries will only increase in the future. For example, the European AIDS Treatment Group collaborates with the Horizon 2020 project EmERGE (2016). Its aim is to develop a mHealth (mobile health) platform to enable self-management of HIV in patients with stable disease. Specific goals include collection and coordination of patient outcomes and health economic data.

35.3 Reflections and Persistent Challenges

There is an increasing leadership at the political level, juxtaposing correctly patient involvement with the sustainability and quality of health systems of the future. There are 'pearls' of good practice where patient involvement is embedded into the body politic of an organisation. Although NICE has been criticised for some dimensions of its work, it is still seen as a 'benchmark' when it comes to involving patients meaningfully. Therein lies a perceived dilemma. Critics argue, why ensure meaningful patient involvement if the decisions are negative at the end of the day? These comments reflect a core misunderstanding. If a robust, inclusive, evidence-based process has been put in place that also genuinely incorporates the patients' perspective, and if transparency and fairness can be demonstrated, then patients, and the wider public, are more likely to accept and understand the decisions that have been made, be they positive or negative.

In the USA, with the emergence of value-based payment arrangements, and 'value models', the National Health Council (NHC), with stakeholder input, has created a *Patient-Centered Value Model Rubric* (NHC 2016). The purpose of this is to provide a tool that the patient community, physicians, health systems and payers can use to evaluate the patient-centredness of value models and to guide value model developers on the effective patient participation throughout their processes. This is a very recent development, and its impact will be evaluated on an ongoing basis. Observers will be keen to see how far such a rubric could be applied in other regions of the world, complementing existing tools and materials.

Reflecting the current political focus at EU level on equitable access to medicines, a new Central and Eastern Europe (CEE) Think Tank on Patient Access was formed in March 2016. The initiative gathers for the first time experts, medical doctors, pharmaco-economists and representatives from patient associations from the CEE region. Utilising a pragmatic multi-criteria assessment approach, the group designed a pilot cross-country HTA process involving Bulgaria, Romania, Hungary, Slovakia,

Croatia and key taskforce stakeholders (scientific experts, decision-making body representatives and governmental officials). The pilot aims to implement this model as a single assessment across countries and a resource-conscious form of HTA.

From the onset, umbrella organisations have balanced their efforts between strengthening the capacity of patients and patients' representatives and advocating for strategic change.

There are major differences in implementation and in the robustness of HTA processes in each country, where diverse evidential and analytical standards are applied. In a number of key disease areas, patient involvement in HTA remains an aspiration, rather than a reality. The fundamental issue of resources remains a challenge as to involve patients optimally requires significant investment. In addition, a blurred definition of what type of patient input and what kind of advisory profile are required (e.g. individual patient, patient advocates or patient advocate experts) renders patient input less effective or targeted.

At the regional and global level, the emergence of new technologies including personalised and targeted gene therapy alongside restricted resources increases the concern among umbrella and patients' organisations that emerging systems may focus on health-economic assessments to the detriment of wider societal costs and benefits. The development of WHO guidance and support to member states has increased the resonance and traction of arguments for greater patient involvement in HTA through its explicit linking of HTA to universal health coverage, health equity and person-centred healthcare.

There is no single 'perfect pathway' to patient involvement in HTA, but the fundamental principles of the right to participate in processes and decisions that will affect patients' lives and for full transparency of decision-making remain. Across the board, national and umbrella patient organisations continue to challenge the development of HTA systems emerging with a narrow focus on cost reduction. Without patient involvement, such HTA processes will remain ill-equipped to deliver value for patients. Value for patients must be evaluated in the context of a holistic perspective that takes into account their quality of life and well-being and capacity to undertake employment and participate in family and community life.

References

ADAPTSMART. Accelerated development of appropriate patient therapies a sustainable, multi-stakeholder approach from research to treatment-outcomes (2016). http://adaptsmart.eu/. Accessed 17 Aug 2016.

EATG. AIDS Treatment project. Press Conference. In: EATN (1996). http://www.eatg.org/wp-content/uploads/2016/05/1996vol5n6.pdf. Accessed 20 Nov 2016.

CORD. Affiliate/Group. http://www.raredisorders.ca/cord-membership/#section-id-157 (2016). Accessed 16 Nov 2016.

EMA. Patients and consumers (2016a). http://www.ema.europa.eu/ema/index.jsp?curl=pages/partners_and_networks/general/general_content_000317.jsp&mid=WC0b01ac058003500c. Accessed 16 Nov 2016.

EMA. Partners and networks (2016b). http://www.ema.europa.eu/ema/index.jsp?curl=pages/partners_and_networks/general/general_content_000212.jsp&mid=. Accessed 15 Sept 2016.
EmERGE. EmERGE mHealth platform. http://www.emergeproject.eu/. 2016. Accessed 07 Sept 2016.
EPF. Health Technology Assessment: European Patients' Forum seminar report, European Patients' Forum. 2010. http://www.eu-patient.eu/globalassets/library/conferenceseminarreports/hta-seminar-2010-brussels-report.pdf. Accessed 15 Sept 2016.
EPF. Patient involvement in Health Technology Assessment in Europe: results of the EPF survey. European Patients' Forum. 2013. http://www.eu-patient.eu/globalassets/projects/hta/hta-epf-final-report2013.pdf. Accessed 16 Aug 2016.
EPF. European Patients' Forum (2016). www.eu-patient.eu. Accessed 15 Sept 2016.
EUPATI. European Patients' Academy on Therapeutic Innovation (2016a). http://www.patient-sacademy.eu/. Accessed 16 Aug 2016.
EUPATI. European Patients' Academy. 2016b. https://www.eupati.eu. Accessed 16 Aug 2016.
European Commission. High Level Pharmaceutical Forum—2005–2008—Final Report. European Commission. 2008. http://www.anm.ro/_/Final%20Conclusions%20and%20Recommendations%20of%20the%20High%20Level%20Pharmaceutical%20Forum.pdf. Accessed 18 Nov 2016.
European Commission. Report on the public consultation on the modalities of stakeholder consultation in the future Health Technology Assessment Network, European Commission. 2012. http://ec.europa.eu/health/technology_assessment/docs/cons_hta_network_results_en.pdf. Accessed 06 Sept 2016.
European Multiple Sclerosis Platform, European register for multiple sclerosis project report 2011–2014. 2016. http://www.emsp.org/wp-content/uploads/2015/06/140930-EUReMS-Report.pdf. Accessed 17 Aug 2016.
EURORDIS. The voice of 12,000 patients—experiences and expectations of rare disease patients on diagnosis and care in Europe. 2009. http://www.eurordis.org/IMG/pdf/voice_12000_patients/EURORDISCARE_FULLBOOKr.pdf. Accessed 17 Nov 2016.
EURORDIS. Access to orphan drugs (2016a). http://www.eurordis.org/content/access-orphan-drugs. Accessed 17 Nov 2016.
EURORDIS, Eurordis Summer School (2016b). http://www.eurordis.org/summer-school. Accessed 15 Sept 2016.
Health Equality Europe. Understanding Health Technology Assessment (HTA). Health equality Europe. 2008. http://img.eurordis.org/newsletter/pdf/nov-2010/58-1%20HEE%20Guide%20To%20HTA%20for%20Patients%20English.pdf. Accessed 15 Sept 2016.
IAPO. International Alliance of Patients' Organizations. www.iapo.org.uk. 2016. Accessed 15 Sept 2016.
Marcus AD. Lessons from AIDS/HIV advocacy efforts. In: Health blog: the Wall Street Journal. 2011. http://blogs.wsj.com/health/2011/06/16/lessons-from-aidshiv-advocacy-efforts/. Accessed 16 Aug 2016.
MedTech Europe. 2015 Priorities of the "Patient-MedTech Dialogue". Focus on Medtech. 2015. http://medical.technology/newsletternews/762/86. Accessed 17 Aug 2016.
NHC. The patient voice in value: the National Health Council patient-centered value model rubric. NHC. 2016. http://www.nationalhealthcouncil.org/sites/default/files/Value-Rubric.pdf. Accessed 18 Nov 2016.
Pivik J, Rode E, Ward C. A consumer involvement model for Health Technology Assessment in Canada. Health Policy. 2004;69:253–68.
SEED. Shaping European Early Dialogues. http://www.earlydialogues.eu/has/. 2015. Accessed 31 Oct 2016.
Wong-Rieger D. Patients as partners in HTA. 2013. http://www.ispor.org/meetings/neworleans0513/releasedpresentations/W20_Wong-Rieger.pdf. Accessed 16 Aug 2016.

Chapter 36
Discussion: Perspective of an HTA Appraisal Committee Chair

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36.1 Introduction

This chapter looks at the three preceding chapters, which describe patient involvement in HTA from the standpoints of healthcare technology developer chapters (Chaps. 33 and 34) and patient organisations (Chap. 35) and reflects on these chapters from the standpoint of HTA bodies. Common themes such as the need for life cycle involvement in new technology development, the need for transparency and the benefits of collaborative working are explored. Patient organisations need support to develop their skills in contributing to HTA; such support should come from multiple sources to minimise any possibility of undue influence or bias affecting the patient view. HTA bodies need to actively work with patient organisations to optimise the relevance of patient inputs to HTA. Additionally, HTA bodies need to tailor their processes to maximise the impact of patient inputs to their assessments and decision-making. My own experience, working in the HTA of new medicines and diagnostic technologies in HTA bodies seeking to promote patient involvement, suggests that such promotion of patient involvement places responsibilities on HTA bodies. I summarise some of these responsibilities, concluding with some personal observations on the 'added value' that patient involvement in HTA can bring.

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36.2 Key Themes from Stakeholder Chapters

36.2.1 Life Cycle Involvement

All three chapters make reference to the increasing involvement of patients in the design, testing, licensing/registration and HTA of new medical technologies. This is clearly welcome and ensures that consideration of patient aspects is not a late addition to other considerations but is integral to the development process. It also means that patient input to HTA is not seen in isolation but as part of wider involvement, perhaps even beginning with participation in nascent 'early dialogue' projects as described in Chap. 35. Time pressures to contribute to HTA reviews of new technologies can also hopefully be eased by earlier and more sustained involvement in health technology development.

Patient input to clinical trial design and assessment should also see increasing use of Patient-Relevant Outcome Measures (PROM), which should facilitate assessment of the real clinical value of new interventions, to the considerable benefit of HTA and also to guide clinicians and patients in their own individual assessments (Turner-Bowker et al. 2016; Botero et al. 2016; Bottomley et al. 2016). While some 'PROMs' are being introduced by enlightened technology developers (Chap. 33), they still very much take second place to the primary and secondary outcome measures assessed in clinical trials. It is encouraging to note that demonstration of 'value', as distinct from 'efficacy' or even 'effectiveness', is perceived as being paramount, and it is to be hoped that this will increase the use and importance of PROMs in coming years.

Indeed, beyond PROMs, there could be considerable value in health technology developers undertaking or commissioning qualitative and quantitative research into wider patient perspectives around the disease area and the new technology. This could be helpful to HTA bodies and help patient organisations wishing to input to HTA assessments by making available some underpinning data to inform their submission.

36.2.2 Transparency

The need for transparency around the interactions between healthcare technology industries and patient organisations is noted in all three chapters, welcoming the real progress made on this theme in recent years (Colombo et al. 2012; Lee et al. 2015). Umbrella patient organisations offer examples of good practice in this regard and can set standards against which individual organisations may be assessed. Such transparency should, of course, apply to all stakeholders and participants in the HTA process, so no different standard should be applied to patient organisations.

Transparency, however, goes beyond matters of finance and sponsorship of patient organisations to include the governance, legitimacy and representativeness of patient organisations. HTA bodies will legitimately ask questions such as—"by what right

does this organisation claim to speak for patients?", "who are the patients for whom it claims to speak?" and "how has it gathered evidence of the views of patients?". The answers to these questions may influence the weight given to patient organisation inputs, with greater legitimacy and representativeness enhancing the impact of patients' perspectives. Manufacturers (Chap. 34) may have difficulty in identifying patient organisations with the appropriate attributes, so patient organisations seeking involvement need to highlight their governance structures and ways of working.

36.2.3 Collaborative Working

All three chapters note the importance of collaborative working, with collaborations within stakeholder groupings (e.g. amongst pharmaceutical companies or patient organisations) and across stakeholder groupings (e.g. industry with patients). Examples such as the Innovative Medicines Initiative (from the industry standpoint) and the International Alliance of Patients' Organizations and European Patients' Forum (from the patient standpoint) show the benefits of pooling resources and sharing experiences, as well as enhancing input to and influence on policy development and decision-making. Older ways of working in which competition (for market share in the case of industry or for share of healthcare expenditure in the case of patient organisations) dominated are no longer so effective in evidence-based, value-focused assessment processes.

36.2.4 Inter-Country Heterogeneity

The very significant differences in approach to HTA (and to patient involvement in HTA) between different countries are noted in all three chapters. This heterogeneity is problematic for technology manufacturers and patient organisations alike and, thus far, has shown little tendency to reduce despite inter-country initiatives such as EUnetHTA. Training and support from international groupings therefore has to be generic, around principles of involvement in HTA, with a need for country-specific training and support, often involving local HTA bodies. As noted in Chap. 34, this is an important role for local HTA bodies, which should actively provide such help rather than expecting patient organisations to navigate complex processes unaided.

36.2.5 Support/Training of Patient Organisations

The promotion of patient involvement in HTA has placed a significant burden of new activity on many patient organisations, which previously were often campaigning or patient support organisations rather than contributors to HTA assessment.

This has necessitated considerable development of individuals within the organisations to allow them to best contribute to HTA processes. While some involvement of HTA bodies in this support and training is appropriate, it is important that external support is also available to avoid the potential for HTA bodies to appear to influence or shape patient opinion.

Many of the excellent 'capacity building' initiatives worldwide are described in Chaps. 33 and 35, some with the support of industry but often at distance from the sponsoring company or as part of a consortium of sponsors. The focus of such support and training should be around enhancing patient input; it is vital to avoid turning patient advocates into 'HTA experts', possibly undermining the unique and valuable patient perspective of their inputs.

36.2.6 Patient Organisation Inputs

As noted in both healthcare technology developer chapters (Chaps. 33 and 34), current patient input to HTA is not simply (or even mainly) factual but also contextual. While submission templates will often seek factual information on the new technology itself, that information is likely also to be available from the manufacturer of the technology, informed by clinical trials or 'real world' data on the use of the new technology. It is the focus on the context provided by the patients' experiences of living with a disease, or the limitations of existing technologies, that is uniquely available from patient organisations. This differentiation between factual and contextual content may reduce over time as patient involvement in technology development occurs earlier in the product life-cycle.

HTA bodies expect that patient input will seek to influence their assessment and appraisal rather than adopting an unbiased view; patient organisations should not be impartial but should advocate on behalf of their constituency. In taking this role, patient organisations need to represent facts and opinions fairly and honestly, but do not need to see all sides of the argument equally. In this regard, they are no different from manufacturers submitting a new technology to an HTA body; the sponsor would be expected to take a positive view of their new technology but also to provide full and honest data and analysis.

36.2.7 Information to Patient Organisations

Chapter 33 notes the continuing difficulties in some jurisdictions for manufacturers in supplying information to patient organisations on new technologies to allow these organisations to contribute to HTA activities, concern being raised that this may be seen as 'direct to patient' marketing or promotion of an unlicensed medicine. Codes of practice (EFPIA 2011; ABPI 2016) and, in some countries, the legal framework inhibit such interactions. However, these restrictions date from a time

when patients were much less actively involved in technology development and assessment.

The last two decades have appropriately seen clinical trial participants receive full (and continuously updated) information on new technologies to allow them to give (and maintain) fully informed consent to their trial participation. If such information can be made available for the purpose of trial participation, then making relevant information available to a patient organisation to allow its participation in the regulatory or HTA assessment of a new technology must be equally appropriate. Codes of practice (and laws) that prevent this are clearly not fit-for-purpose in the context of new models of technology development and urgently need to be revised and updated; the chapter gives examples of good practice in this area.

36.3 Roles and Responsibilities of HTA Bodies Working with Patients

HTA bodies seeking to involve individual patients in their work and/or to obtain submissions of information on new technologies from patient organisations must recognise that they need to be active stakeholders and not simply passively await inputs. They also need to recognise that they have responsibilities toward the patients and patient organisations with whom they are working (Abelson et al. 2016; Rashid et al. 2016; Low 2015).

36.3.1 Patient Organisations

36.3.1.1 Information that is Helpful in Assessment/Appraisal

As noted in the previous section, involvement in HTA is a significant new burden on many patient organisations, often in the face of significant resource constraints. It is therefore important for HTA bodies to give very clear advice about what information will usefully contribute to the HTA process and how it should be presented, to maximise its impact while minimising the workload on the patient organisation. Simply inviting patient organisations to provide any information they wish is unhelpful. While patient organisations must be able to make the inputs they wish, help in focusing or targeting their submission is highly appropriate.

36.3.1.2 Process Considerations for Patient Input

Patient submissions to an HTA process must enter the process at a point at which there is still significant equipoise around the final decision, such that the patient input can have significant impact. This will usually mean early in the process as part

of initial assessment of other evidence. Reviewing all clinical data and data on cost-effectiveness, for example, and only then factoring in patient input is not appropriate.

36.3.1.3 Impact of Patient Input

Patient organisations often feel that the impact of their efforts is little noted in outputs from HTA bodies, leaving them feeling undervalued. At a minimum, HTA bodies should, in their outputs, describe the patient input(s) received and considered; ideally, there should also be some comment on the influence that such inputs had into the outcome of the assessment.

36.3.1.4 Patient Input if Final Assessment Negative

HTA bodies need to be very clear that, where an assessment/appraisal leads to a decision not to introduce or fund a new technology, it is the technology that has failed the assessment, not the patient input that has failed. Patient organisations must not be left feeling that they have failed their members if a technology is not approved, while members should not be left feeling that their patient organisation has let them down. Some patient organisations are already considering disengagement from HTA input as they feel that they are left to take the blame—this helps neither patients nor HTA bodies, so it is in the interest of everyone to show the value of patient input, even if it has not led to approval of the technology.

36.3.2 Patient Members of HTA Bodies

36.3.2.1 Roles and Responsibilities

Many HTA bodies are now involving patients as full members of assessment/appraisal committees, having their voice (and often their vote) at the decision-making table. The primary role of such individuals is to ensure that the needs and interests of patients remain at the heart of HTA assessment, appraisal and decision-making. There is a legitimate concern that the needs and views of others in the healthcare system (insurers, other payers, healthcare providers, etc.) may begin to take precedence, so patient members of committees have an important role to ensure that ‘due process’ and patient focus are maintained (Panteli et al. 2015).

Patient members may also have an important role in presenting and discussing inputs from patient organisations in committee discussion and deliberation. This role may require specific training (see below). Good practice should dictate a minimum of three patient members of HTA bodies with the aim of at least two being present at each meeting to avoid a patient member ever being a ‘lone voice’.

36.3.2.2 Support and Training

Clearly patient members of HTA committees need training in their roles and ongoing support. While some of this will come from fellow patient members of the committee, it is appropriate that the HTA body itself provides some training and support. The aim of the training is to support the different perspectives that patients bring to the HTA process, not to create experts in HTA who are, in effect, indistinguishable from other members of the committee. Careful and sensitive consideration of the needs of patient members and how they may best be met are therefore needed.

36.3.2.3 Avoidance of Blame

Patient members of HTA decision-making bodies must be protected from any feelings that they are individually to blame for any decision not to approve a new technology. The feelings of blame may be internal (the patient member blaming themselves) or external (patient organisations or individual patients blaming patient members for failing to get the technology approved). Both are wrong as the HTA process has so many inputs that no one input is responsible for either a positive or negative decision, but patient members may need some support, and even defence, if they are unfairly targeted as being to blame for a negative decision.

36.4 Reflections of an HTA Appraisal Committee Chair

HTA bodies therefore have an active role in the process of involving patients in their deliberations, and need to work carefully and supportively with patient members and patient organisations to maximise the benefits and impact of their involvement while maintaining sufficient distance to avoid undermining (or even appearing to undermine) their independence and distinctive views on issues.

As an HTA appraisal committee chairman and experienced health professional, I really appreciate the added value that a patient submission, and patient involvement in decision-making, brings to the process. Primary and secondary endpoints in a clinical trial (of a new medicine or other technology) almost never tell the whole story of the adverse impact of any disease, and therefore changes in these endpoints equally rarely describe the full impact of new interventions. A patient organisation can more fully describe the impact and ramifications of the disease and the limitations of current interventions, and can go beyond strict trial endpoints in showing possible benefits (and sometimes even drawbacks) of new technologies. In making decisions about approval that will affect patients, these wider insights reassure me that the committee is fully aware of the context and implications of the decision that it is making.

The presence of patients in the actual decision-making process is, for me, a constant check that outcomes and benefits for patients are at the heart of the process,

helping me as chair to focus discussion and debate on patient-relevant issues. In addition, I believe that having patients at the decision-making table helps legitimise decisions, even where these are to not approve the introduction of new technologies. It is easy to caricature clinicians and other professionals involved in HTA activities as 'heartless bean-counters' or as knowing 'the price of everything and the value of nothing' (to quote Oscar Wilde in 'Lady Windermere's Fan'); real patients are not so lightly dismissed!

From my personal experience over the last 15+ years, patient involvement in HTA comes with few, if any, insuperable drawbacks from the standpoint of any interested stakeholder and brings real added-value to decision-making, improving the decisions to the benefit of all concerned.

References

Abelson J, Wagner F, DeJean D, Boesveld S, et al. Patient and public involvement in health technology assessment: a framework for action. Int J Technol Assess Health Care. 2016;32:256–64. doi:10.1017/S0266462316000362.

ABPI. Code of practice for the pharmaceutical industry. 2016. http://www.abpi.org.uk/our-work/library/guidelines/Documents/code_of_practice_2016.pdf. Accessed 25 Nov 2016.

Botero JP, Thanarajasingam G, Warsame R. Capturing and incorporating patient-reported outcomes into clinical trials: practical considerations for clinicians. Curr Oncol Rep. 2016;18:61. doi:10.1007/s11912-016-0549-2.

Bottomley A, Pe M, Sloan J, Basch E, et al. Analysing data from patient-reported outcome and quality of life endpoints for cancer clinical trials: a start in setting international standards. (Setting standards in analyzing patient-reported outcomes and quality of life endpoints data (SISAQOL) consortium). Lancet Oncol. 2016;17:e510–4. doi:10.1016/S1470-2045(16)30510-1.

Colombo C, Mosconi P, Villani W, Garattini S. Patient organizations' funding from pharmaceutical companies: is disclosure clear, complete and accessible to the public? An Italian survey. PLoS One. 2012;7:e34974. doi:10.1371/journal.pone.0034974.

EFPIA. Code of practice on relationships between the pharmaceutical industry and patient organisations. 2011. http://transparency.efpia.eu/uploads/Modules/Documents/code_po2011.pdf. Accessed 25 Nov 2016.

Lee A, Jones J, Brown A, Macfarlane K, Fox JG. Increasing transparency and the patient voice in HTA of new medicines. Value Health. 2015;18:A561–2. doi:10.1016/j.jval.2015.09.1829.

Low E. Potential for patients and patient-driven organizations to improve evidence for health technology assessment. Int J Technol Assess Health Care. 2015;31:226–7. doi:10.1017/S0266462315000434.

Panteli D, Kreis J, Busse R, Int J. Considering equity in health technology assessment: an exploratory analysis of agency practices. Int J Technol Assess Health Care. 2015;31:314–23. doi:10.1017/S0266462315000549.

Rashid A, Thomas V, Shaw T, Leng G. Patient and public involvement in the development of healthcare guidance: an overview of current methods and future challenges. Patient. 2016;1:1. doi:10.1007/s40271-016-0206-8.

Turner-Bowker DM, Hao Y, Foley C, Galipeau N, et al. The use of patient-reported outcomes in advanced breast cancer clinical trials: a review of the published literature. Curr Med Res Opin. 2016;32:1709–17. doi:10.1080/03007995.2016.1205005.

Chapter 37
Reflections for Future Development

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37.1 Introduction

This chapter reflects on some key themes emerging in this book. The book provides a starting point for HTA bodies and academics to consider, research, develop, pilot and implement patient involvement processes in collaboration with patients and other stakeholders. The first step is to reiterate what we mean by patient involvement in HTA, then give considerations of the why, how, when and who is involved. The 'what' has been established in previous HTAi work defining patient involvement as including (1) research into patients' experiences, perspectives and preferences and (2) patient participation in the HTA process. The 'why' of patient involvement is clarified by new research that identifies high-level goals for patient involvement in HTA as democratic, developmental, instrumental and scientific. Authors from diverse stakeholder groups explain mechanisms for patient involvement that arise from long-standing and emerging research methodologies and patient participation processes. Considering these methodologies in light of the

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rationales and goals for an individual, HTA helps to elucidate the ‘how’, ‘when’ and ‘who’ of patient involvement for HTA.

37.1.1 Clarifying Concepts

The consideration of ‘what’ HTA is is not straightforward given differences in terminology internationally, particularly in relation to the keywords of ‘involvement’ and ‘HTA’. The diverse nature of HTA was explored in Chap. 1, as was the definition of involvement used in this book (Facey et al. 2010), but further elucidation of the concepts within involvement were needed as the book developed.

The processes that enable patients and patient groups to provide written or verbal contributions to HTA were defined as ‘patient input’ (borrowing terminology from CADTH). Chapter 6 was added to the book, which identified patient input as the subset of participation mechanisms where the patient or patient representative must take the initiative to contribute specific structured information to the HTA process via consultation processes, via submission templates or in multi-stakeholder meetings. As patient input was clarified, so too was the description of research outputs as patient-based evidence (Chap. 4).

The differentiation between the forms of research required to understand patients’ perspectives, experiences and preferences was explored in Chaps. 3, 12, 15 and 17. It was clarified that research about patients’ experiences of a condition or use of a health technology requires a different research construct than the research required to study patients’ preferences, changing from a configurative to an aggregative framework (Chap. 17). Furthermore, the generalisability of this research will be different, with experiences of a condition applicable across several HTAs, but preferences being health technology and population or culture specific. More debate on these constructs is needed with researchers who can lead us into a better understanding of knowledge and epistemological frameworks.

37.2 Goals of Patient Involvement in HTA

Over the past decade, there has often been a lack of clarity about the goals of patient involvement in HTA. This means that the most critical question in relation to patient involvement in HTA is ‘why?’. This may be answered by the phrase Gauvin et al. (2010) [1518]) used to explain variations in the way the HTA community conceptualises patient involvement, ‘it all depends’. ‘It depends’ on the goals and context of the HTA process and the specific questions addressed in an individual HTA.

Although we can say that the general goal of HTA is to undertake a systematic, scientifically based process to inform policy, the process of HTA is unique to each HTA body (Chap. 1) and may vary according to the type of health technology being assessed. HTA is shaped by the political context in which it sits, the construct of the health-care systems it seeks to inform, its legal basis, the scientific evidence that can

Table 37.1 Theoretical goals of (public and) patient involvement in HTA (OHTAC 2015)

Theoretical goal	Description
Democratic	Achieving more informed, transparent, accountable and legitimate decisions about health technologies
Developmental	Increasing public understanding of health technologies and HTA and strengthening the public's and patients' capacity to contribute to health technology policy issues
Scientific	Promoting a more robust and comprehensive approach to HTA that incorporates social values and ethics, as well as patients' problems, lived experiences, outcomes and preferences
Instrumental	Making better-quality decisions across all stages of the HTA process

be accessed, the expertise available to assess the scientific evidence and other historical precedents such as whether relations with stakeholders are encouraged or discouraged. Consequently, the context and goals of an HTA body must be understood before its goals for patient involvement can be established.

A recent literature review identified goals of patient (and public) involvement in HTA (Table 37.1). These theoretical goals were used to delineate the goals for patient involvement at various stages of the HTA process in Ontario, and then specific mechanisms of involvement were identified to match the goals (Abelson et al. 2016).

It could be argued that all HTA bodies should promote patient involvement to support the scientific and instrumental goals stated in Box 37.1. This would help ensure that the best evidence and information about the impact of the health technology on patients' lives is available throughout the HTA process and can inform the wide range of value judgements and decisions that are required. The democratic and developmental goals identified in Box 37.1 might be particularly important for HTAs that directly inform health technology reimbursement decisions, to demonstrate accountability.

If the goals of patient involvement in an HTA are unclear, this could lead to processes and methodologies that do not provide value to any stakeholder in the HTA process. The requirement is not for all HTA bodies to have the same goals for patient involvement in HTA, but for each to be explicit about their own goals. Patient involvement can then be shaped appropriately with all stakeholders and evaluated according to the goals that have been set.

37.3 Research and Participation

37.3.1 Balancing Research and Participation

This book presents a range of research methodologies that can be used to study patients' perspectives, experiences and preferences and a variety of mechanisms to support patient participation in HTA. Participation strongly supports the 'democratic' and 'developmental' goals outlined in Box 37.1, especially when participation is undertaken in a way that supports dialogue. Whereas research to produce

Box 37.1 Areas of Decision Uncertainty to which Patients can Contribute (Menon et al. 2015)

Clinical benefit

(a) Are the benefits observed in the trial generalisable to the patient population within the relevant jurisdiction?
(b) Does the level of health gain observed vary across patient subtypes?
(c) Which outcomes should be measured?
(d) What is the natural progression of the disease?
(e) What is known about the effect of the drug compared to that of current best practice?
(f) What is the meaningfulness to patients of the health gain attributable to the drug?

Value for money

(g) What are the broader implications associated with the drug, beyond clinical benefit?
(h) What opportunity costs are associated with funding the drug?
(i) What is known about society's willingness to pay for the expected gain?

Affordability

(j) How many patients are expected to benefit from the drug?
(k) What is the expected cost per patient per year?

Adoption/diffusion

(l) How will access to the drug be managed?
(m) Who has the expertise to decide on starting and stopping criteria?
(n) Are mechanisms compelling patients and physicians to ensure appropriate use required?
(o) Are there other drugs in the pipeline that may affect utilisation of this drug in the near future?

patient-based evidence may be seen as achieving the 'scientific' goal, both research and participation support the 'instrumental' goal.

As the case studies in Part III demonstrate, most HTA bodies focus on patient input, with limited use of patient-based evidence. This may be due to government policies that promote patient participation in designing healthcare services and demands from patients to be included as a democratic, instrumental or basic human right. The paucity of research to produce patient-based evidence for HTA may also be

a result of time and cost factors. With limited budgets and increasing use of rapid HTAs, patient input is often used as a proxy for patient-based evidence. This approach potentially devalues patient-based evidence and patient input by failing to recognise their complementary functions in an HTA. It risks using each inappropriately, leading to questions about the robustness of patient-based evidence and diminishing the potential for the responsiveness and shared learning of participation.

Evidence must be critically scrutinised by researchers and clinicians with the knowledge gained weighed against the other inputs in the deliberative processes that lead to HTA recommendations (Facey and Hansen 2011). Part of the challenge we see in HTA is that the balancing of knowledge and evidence sources is not explicit. Facey et al. (2010) recommended that HTA reports contain a specific section about patients' perspectives. We go further and say that this section should clearly describe the goal of patient involvement in the HTA, how patients have been involved, the key insights that were gained from them and how these insights impacted the deliberative discussions that led to the HTA recommendations.

While the separate but complementary nature of research and participation has been emphasised in this book, in practice the two areas can interact. We identify that there is a need for further careful reflection on the distinction this book makes between patient-based evidence and patient input and the value and limitations of both research and participation processes.

37.3.2 Participation

Processes for patient participation are more popular in HTA, but if reduced to one-way communication, e.g. using only written submissions, the potential for dialogue, responsiveness, problem-solving and capacity building is minimised or lost. Additionally, for patient input to be credible and legitimate, there is a need for greater awareness and consideration of the value and limitations of using patients or patient representatives as 'experience-based experts' (Boivin et al. 2014, Chap. 3). Participating in an HTA often requires a level of competence (to understand scientific papers, engage confidently in debate, etc.) and physical stamina. Hence, HTA bodies and patient organisations often go to great lengths to identify patients that can participate in HTA processes and train them to do so. For this reason, patient representatives may participate, rather than or alongside individual patients. The implication of focusing participation processes on inviting patients and patient representatives into HTA processes and meeting places, rather than taking processes and meetings to patients, is an area identified for further work. The merits of the different mechanisms of participation suggested in the mosaic in Chap. 5 need to be debated.

37.3.3 Research

This book has stressed that HTA is interdisciplinary and should include research from the social sciences, not just from clinical research and economics. While major academic developments have been seen in economic modelling for HTA, research to produce patient-based evidence has not been similarly championed. This may be due to misconceptions by HTA professionals, grounded in quantitative clinical research methods, that research to produce patient-based evidence is 'unscientific' or of poor quality (Chap. 4). Social science offers flexible research designs that can provide rich, holistic descriptions of a complex situation. Its stringent methodologies tackle concerns about bias, by requiring reflection on patients' and researchers' subjectivity (Chap. 17) with clear presentation of the research limitations. Such research can place less burden on patients and their representatives than input processes and can be critically appraised. It can also include individuals that might not respond to contact from patient organisations or make their own submissions to an HTA—thus opening a communication channel with those who are seldom heard. In this way, the burden on patients as 'experts' in input processes is shared or reduced, and the representatives can be supported with complementary research when they participate, as they are still likely to want 'a seat at the table'. While this book demonstrates a wide variety of research methods to study patient aspects, their uptake depends on addressing present misconceptions, increasing awareness of their value and investing in skilled professionals in this field in the HTA community.

In writing this book, we recognise that the *HTAi Values and Quality Standards for Patient Involvement in HTA* are focused on the issue of participation. The issues of Quality Standards for patient-based evidence are not covered, and this is a critical gap that needs filling. Although research has its own standards, there would be merit in being more explicit about Quality Standards for patient-based evidence given the range of stakeholders involved in such research; standards may help to explicitly promote its rigour.

37.4 Patient Involvement to Inform Deliberations About Value

To ensure best use of the resources of patient groups and HTA bodies, consideration should be given about when patient involvement adds most value in relation to the goal(s) determined for an individual HTA. When clinical and economic evidence is unclear, patient involvement can provide important insights about local care delivery, experience with current therapies and patient benefit. Furthermore, when the effectiveness of a health technology depends on the patient's ability or willingness to use it, patients' experiences would seem essential. In HTAs based on clinical and cost-effectiveness, where a health technology has a cost/QALY much higher than a specified willingness-to-pay threshold, it is questionable whether patient involvement in

HTA can make a difference. If it does not, there is a danger that patient involvement wastes efforts and undermines relationships by creating unrealistic expectations.

Menon et al. (2015) have studied the HTA systems that make reimbursement decisions and offer specific examples of when patients can provide instrumental input to HTA. They consider the following questions about decision uncertainty.
The questions set out by Menon et al. (2015) provide a useful starting point for discussion among HTA committees and patient groups to identify if these are all the areas where patient involvement is of most benefit. Processes could then be shaped to ensure patient involvement can contribute to these areas of uncertainty.

Patient involvement in HTA has often been conceived as beginning at topic selection; however, Chaps. 33 and 34 indicate that patient involvement can begin in the development of health technologies. Health technology developers now commission a range of quantitative and qualitative research studies about patients' perspectives. These have been mainly undertaken for internal purposes to guide clinical development plans or for marketing purposes, and they have rarely been published. Publishing this research could avoid waste and help to optimise the use of resources. The issue of credibility and bias is also often raised when health technology developers publish such research or submit the research as part of an HTA dossier. Like the other forms of evidence the developers provide, this research can be critically appraised according to checklists developed specifically for this form of evidence (Lewin et al. 2015; SBU 2013; Bridges et al. 2011). Developing rigour in this research can make it a valuable part of the evidence base in an HTA submission.

37.5 Collaborative Working

The authors of the chapters in this book come from a wide range of perspectives, disciplines and organisations. We must work together in thoughtful collaboration, building on each other's ideas and knowledge to create frameworks of patient involvement in HTA that deliver a measurable impact to HTA.

Gauvin et al. (2011) noted that HTAi and INAHTA have developed close-knit international collaborations to develop and promote HTA, but that they could do more to legitimise and promote patient and public involvement in HTA. Over the past decade, experience in the HTAi Interest Group has shown the value of multi-stakeholder collaboration. Synergy is created by honest sharing of challenges and emerging processes so that new ideas can be explored, developed and implemented. In developing and researching this book, the power of this collaboration has become more obvious to us. We have found that collaboration is the magic—enabling those 'light bulb moments' where someone from a particular discipline suddenly realises that there is a whole host of thinking and concepts that they had not considered before.

We would very much like this book to become a catalyst of collaboration across HTA agencies, across disciplines, across nations and across all the actors on the HTA stage. It is through the act of collaboration that trust and relationships are built, common understanding emerges and shared goals can be achieved.

37.6 Conclusion

The HTA processes developed three decades ago recognised that health technologies have implications for patients, their caregivers and society that extend far beyond their effectiveness and safety. The HTA community has grown adept at expressing the value of health technologies in terms of clinical and cost-effectiveness, yet we still struggle to understand their value to patients and caregivers and the implications beyond the health services facilities' doors. HTA includes many professionals with experience in clinical research, health economics and health services research and has used those disciplines to critically appraise evidence to determine the value of a health technology. HTA now needs to embrace a wider field of researchers who can help the HTA community understand and develop patient-based evidence and to recognise patients and their representatives are 'experience-based experts' who have a right to participate in an HTA process.

To continue to improve patient involvement, we need to remember the scientific basis and interdisciplinary nature of HTA and encourage all stakeholders to use their expertise to work collaboratively. We ask:

1. Patient groups to work with all stakeholders to develop patient involvement processes that improve the quality of research and HTA
2. Academics to develop methodologies (including rapid qualitative evidence synthesis, social media research) which can meet HTA timeline requirements, to explain the value of their research and to push to be involved in HTA
3. Health technology developers to incorporate patient involvement in their technology development programmes using sound methodologies and report on this work transparently
4. HTA bodies to define their goals for patient involvement, develop and evaluate their processes for patient involvement and implement the HTAi Values and Quality Standards for Patient Involvement in HTA (Chap. 1)

Our vision is for all the actors on the HTA stage to come together to strengthen their deliberative processes, broaden their range of inputs and share their research and experience on patient involvement, input and evidence. We have a tremendous opportunity to shape the future of HTA for the better by integrating the perspectives, experiences and evidence from the patient communities.

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NB is an independent consultant who undertakes unpaid work as Chair of the HTAi Interest Group for Patient and Citizen Involvement in HTA as well as for other multi-stakeholder coalitions. In these unpaid activities, he sometimes receives expenses to attend meetings. Neil also undertakes consultancy work for the pharmaceutical industry that is paid and may relate to HTA submissions and patient involvement strategies in medicine development.

References

Abelson JA, Wagner F, DeJean D, Boesveld S, Gauvin FP, et al. Public and patient involvement in health technology assessment: a framework for action. Int J Technol Assess Health Care. 2016;32:1–9. doi:10.1017/S0266462316000362.

Boivin A, Lehoux P, Burgers J, Grol R. What are the key ingredients for effective public involvement in health care improvement and policy decisions? A randomized trial process evaluation. Milbank Q. 2014;92:319–50.

Bridges J, Hauber AB, Marshall D, Lloyd A, Prosser LA, Regier DA, et al. Conjoint analysis applications in health—a checklist: a report of the ISPOR Good Research Practices for Conjoint Analysis Task Force. Value Health. 2011;14:403–13.

Facey K, Boivin A, Gracia J, Hansen HP, Lo Scalzo A, Mossman J, et al. Patients' perspectives in HTA: a route to robust evidence and fair deliberation. Int J Technol Assess Health Care. 2010;26:334–40.

Facey K, Hansen HP. Patient-focused HTAs. Int J Technol Assess Health Care. 2011;27:273–4. doi:10.1017/S0266462311000572.

Gauvin F-P, Abelson J, Giacomini M, Eyles J, Lavis JN. "It all depends": conceptualizing public involvement in the context of health technology assessment agencies. Soc Sci Med. 2010;70:1518–26.

Gauvin FP, Abelson J, Giacomini M, Eyles J, Lavis JN. Moving cautiously: public involvement and the health technology assessment community. Int J Technol Assess Health Care. 2011;27:43–9. doi:10.1017/S026646623100012 00.

Lewin S, Glenton C, Munthe-Kaas H, Carlsen B, Colvin CJ, Gülmezoglu M, et al. Using qualitative evidence in decision making for health and social interventions: an approach to assess confidence in findings from qualitative evidence syntheses (GRADE-CERQual). PLoS Med. 2015;12:e1001895. http://journals.plos.org/plosmedicine/article?id=10.1371/journal.pmed.1001895. Accessed 12 Dec 2016

Menon D, Stafinski T, Dunn A, Short H. Involving patients in reducing decision uncertainties around orphan and ultra-orphan drugs: a rare opportunity? Patient. 2015;8:29–39. doi:10.1007/s40271-014-0106-8.

OHTAC Public Engagement Subcommittee. Public engagement for health technology assessment at Health Quality Ontario—final report from the Ontario Health Technology Advisory Committee Public Engagement Subcommittee [Internet]. Toronto: Queen's Printer for Ontario. 2015. http://www.hqontario.ca/Portals/0/documents/evidence/special-reports/report-subcommittee-20150407-en.pdf. Accessed 27 Aug 2016.

SBU. Evaluation and synthesis of studies using qualitative methods of analysis. 2013. www.sbu.se/handbook. Accessed 12 Dec 2016.

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