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COMMENTARY

Giving Patients a Meaningful Voice in European Health Technology Assessments: The Role of Health Preference Research

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1 Introduction

Throughout Europe, formal health technology assessments (HTAs) are increasingly being required for regulatory decision making. Although the institutional and legal contexts for HTA vary by country, HTA typically involves evaluations of causal evidence and requires assessing tradeoffs among multiple clinical trial endpoints and multiple, often conflicting objectives. Although the assessment requires evaluating the quantity and quality of evidence, decision makers do apply values at some point that attach weights to multiple decision criteria from multiple perspectives. Thus, the outcomes of decision processes depend both on their identified endpoints and on the relative importance attached to the decision criteria (i.e., the decision weights) [1].

Values can be defined from different perspectives: those of the payers, the industry, the clinicians, and, increasingly, the patients. While HTA can be regarded as a process for systematically applying values to treatment attributes to allocate scarce resources, which values to apply and how each perspective is incorporated are not clearly communicated by most European HTA agencies, revealing little about experts' priorities or the role of patients' concerns.

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The opacity of HTAs serves the self-interest of decision makers by facilitating the use of their own strategic values and averting disputes over the importance of alternative perspectives. By merely stating conclusions without revealing the decision weights attached to various endpoints or perspectives, decision makers retain the freedom to apply arbitrary decision weights. These weights need not satisfy standards of internal consistency and relevance or even represent the consensus among decision makers themselves. In the absence of transparency, whether decision weights account for patient priorities (or the priorities of any stakeholder) is unknowable. In this article, we review the role of patient preference evidence in the European HTA processes, describe how the emphasis on patient preferences has caused a methodological shift toward multi-criteria decision analysis (MCDA), and discuss the next steps for enhancing regulatory decision making.

2 Why Include Patient Preference Evidence in Multi-Criteria Decision Analyses?

Patient welfare is presumably an (if not the primary) objective of providing healthcare services. Hence, patient values should logically play a central role in approval, utilization, reimbursement, and pricing decisions. In the absence of evidence regarding patients' preferences to inform the relative weighting of decision criteria, it is difficult to judge the consistency of regulatory decisions with patient values. Therefore, there must be a mechanism for generating and applying patient preference evidence within the MCDA of HTA agencies, particularly in Europe where socialized medicine predominates [2].

In Europe, there are a number of mechanisms for capturing the input of patients to inform regulatory bodies. The benefit-risk methodology report of the European Medicines Agency (EMA) that was produced in 2010 suggests that combinations of approaches characterized by at least two of the following conditions could prove useful: the magnitude of favorable effects, the seriousness of unfavorable effects, uncertainty about the effects, transitions in health states and the time spent in each state, and tradeoffs among effects [3]. Moreover, the EMA has discussed MCDA techniques, including cost-effectiveness analyses, as a way to overcome some of the problems in making benefit-risk assessments for authorization decisions [3].

In addition to the benefit-risk methodology project, the EMA has also supported projects that evaluated alternative methods for incorporating the patient voice in the decision-making process. The "Value and Utilities among European Patients" study was conducted to assess the use of the "Measuring Attractiveness by a Categorical Based Evaluation Technique" to measure patient preferences for treatment outcomes. The "Pharmacoepidemiological Research on Outcomes of Therapeutics by a European Consortium" project was coordinated by the EMA and conducted by a large public–private European consortium and the Innovative Medicines Initiative [4]. Through this work, several methods for eliciting preferences were evaluated, including discrete choice experiments (DCEs) [5]. The preference evidence that was gathered herein informed quantitative benefit-risk modeling exercises to help decision makers construct the values required for MCDA [4]. The project concluded that no single preference-based method can fully capture all aspects of a benefit-risk assessment. However, the selection of a single approach or a combination of methodologies should be matched to the complexity of the problem.

3 Multi-Criteria Decision Analyses Can Integrate Patient and Societal Values

Various methods have been developed to formalize the decision-making processes of regulatory agencies. Some researchers use the term MCDA to include a wide range of approaches, such as collecting large samples to quantify preferences and judgments (e.g., surveys). Ciani and Taylor [6] define MCDA as a "set of methods that seek to score, weigh and ultimately aggregate the various criteria into an overall composite measure of benefit". According to Marsh and colleagues [7], furthermore, MCDA can be used in a variety of decisions, such as the "investment-prioritization of interventions for coverage or reimbursement; prescription-selection of intervention; authorization-assessment for licensing; and research funding-allocation of research funds".

Multiple HTA agencies recommend the use of MCDA as a complement to cost-effectiveness analysis evidence. Like MCDA, cost-effectiveness analysis is a formal decision analytic approach, but it summarizes evidence from a single perspective (e.g., societal or payer) and may be completed by a single analyst. Multi-criteria decision analysis is more of a group-engagement process (in that it involves multiple stakeholders) and is especially useful if complex decisions have to be made that involve multiple divergent perspectives with potentially conflicting decision criteria (e.g., tradeoffs). Dolan [8] states that MCDA is "helpful when there is a need to combine 'hard data' with subjective preferences, to make tradeoffs between desired outcomes, and to involve multiple decision-makers". Multi-criteria decision analysis can be seen as a framework for transparently integrating clinical findings, preference evidence, and expert judgments into clinical and regulatory decision making.

In Germany, the Institute for Quality and Efficiency in Health Care has realized that MCDA procedures may support the weighting of criteria from various efficiency frontiers and has recently tested two procedures (DCEs and analytical hierarchical processes) in pilot projects [9, 10]. A recent case study calculated an efficiency frontier that combined preference data, which was taken from a prior DCE study, and clinical data [11]. The preference data could also be derived from best-worst scaling studies [12].

In Great Britain, the National Institute for Health and Care Excellence has explicitly defined information by the type, format, and sources of evidence in its guidelines for the assessment and testing of eligibility of MCDA in the assessment of interventions. The National Institute for Health and Care Excellence considers patient surveys and patient or expert opinions [13, 14] as evidence. In contrast to the assessment phase, the appraisal of eligibility for reimbursement is usually based on expert opinion, while citizens characterize the societal perspective in response to questions of distributive justice. The move toward MCDA is welcomed by the National Institute for Health and Care Excellence, but the success of this transformation will depend, in part, on the quality of the preference evidence.

Public and patient engagement has long been unstructured in Belgium. Societal and patient values were rarely represented and recognized in the HTA process. Hence, the Belgian Healthcare Knowledge Centre focuses on an increased participation of patients and citizens in decision making. In 2014, the results of its initiative on "Incorporating Societal Preferences in Reimbursement Decisions—Relative Importance of Decision Criteria According to Belgian Citizens" were published [15]. The report discusses several approaches to MCDA and preference elicitation. The authors conclude that no single technique stands out in all respects as the best approach for measuring

preferences regarding reimbursement criteria. However, they state that DCEs are the most advantageous because they require respondents to consider several criteria at once, similar to the process of making decisions in real life.

In Europe, the first step to incorporating the patient voice is recognizing the importance of the patient perspective in regulatory decision making. By incorporating quantitative evidence on patient preferences within an MCDA process, regulators can formally give patients a meaningful voice in HTAs and regulatory decisions. This shift toward patient centeredness has the potential to fundamentally change the development, support, and delivery of healthcare across Europe.

4 Next Steps to Enhance Regulatory Decision Making

Regulators are under continuous pressure to ensure that their decisions are aligned with patient needs. To be responsive to these concerns, experts must have access to evidence on patient preferences and integrate these preferences with the preferences of other stakeholders and with clinical evidence. The implementation of evidence-based medicine requires the best external evidence, individual clinical expertise, and patients' preferences. The integration of patient-preference evidence within European HTA processes will promote its collection as well as the congruence of regulatory decisions with patients' values.

Despite this conclusion, HTA in Europe generally continues to focus on assessing clinical effects and cost effectiveness rather than patients' values. Conventional submissions of clinical evidence do not meet the informational requirements needed to make a rational decision based on patients' preferences as crucial stakeholders. Measurements of clinical effects and societal values continue to be necessary, but are not sufficient to inform regulatory decisions. Patients' lives are not only affected by clinical endpoints, but also by whether their concerns have been formally taken into consideration. This is a matter of respect, not just welfare.

With highly complex issues, multiple stakeholders, and numerous conflicting criteria, the assessment of patient preferences and their incorporation into appraisal frameworks is the first step toward giving patients a meaningful voice in European clinical and regulatory decision making. If patient benefits are seen as the primary output of a health intervention, HTA agencies should motivate, accept, and consider patient preference evidence as a part of its reporting standards. With the support of this evidence, HTA reports may soon include sections that describe the effects of different treatment alternatives in terms of patients' benefits from their own perspectives.

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Compliance with Ethical Standards

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