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Editorial

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The fourth hurdle as controlling instrument for expenditure on medication?

In recent decades, expenditure for the health system has increased unabated in Germany, as in all industrial countries. Although in the end, the amount of such expenditure depends on the willingness of society to pay for its health, this development is accompanied by considerable public criticism. Increased attention is also given to this topic in view of the fact that the rate of escalation in health expenditure clearly exceeds that of the gross national product in the corresponding countries. Whereas during the 1960s health expenditure accounted for about 4% of the gross national product, today it accounts for about 10%. Together with demographic reasons, the causes for such an increase in expenditure are generally said to be the increasingly demanding attitude of patients, increases in prices for medication, and above all, medical progress. Expenditure on medication is frequently a focus of public discussion on the development of expenditure in the health system. In Germany, medication expenditure is the third largest item after in-patient and out-patient costs, accounting for around 16% of total expenditure on health costs by the health insurance funds and companies [8]. The dynamic development of expenditure on the medication sector provides at least a partial explanation for the rate of escalation in total expenditure on health costs.

Other reasons why expenditure on medication is frequently a focus in the discussion on curbing cost expansion include the high data transparency here compared to other sectors, the possibility of central control and regulation which does not exist in other sectors because of self-administration and federal structures, and political motives. Regarding the latter, the regulations on the pharmaceutical industry are supposed to be more acceptable to society at large than those affecting the hospital sector [25].

The stated reasons for the growth in expenditure on medication, in particular medical progress, become apparent primarily in the structure component (e.g. the shift towards prescribing more expensive medication). In Germany, the average prescription value has increased by about 60% over the last ten years, compared to a decline in the actual prescription quantity figures (Medication Prescription Report 2001). Another noticeable fact is the evolution of the average prescription value of medication with new substances. The average prescription value of all new medication introduced since 1986 exceeds the average prescription value of the overall market by around 120% (Medication Prescription Report 2001).

In the past, a large number of statutory regulations have been introduced with the aim of controlling the dynamic development of expenditure on medication. Previous instruments concerned primarily the price and quantity vector of medication expenditure; apart from negative lists, as in Germany for example, product-related criteria are relatively rare. Instruments for curbing cost expansion on medication such as state intervention in terms of pricing, the establishment of reference-pricing, the introduction of co-payments and the compiling of positive or negative lists, have not had the desired effect [18].
In particular, budgets as state controlling instruments on the demand side have not produced the wanted effect of reacting adequately to the different factors influencing the dynamic development of expenditure. This is why the cost effectiveness or value for money of medication has been given increasing attention over the last ten years as a possible controlling and regulatory instrument.

All controlling and regulatory measures aiming to reduce expenditure on medication are to be considered formally as instruments of rationing. Over and above the discussion about using value for money as a decision-making criteria in terms of medication, it is therefore necessary to initiate a dialogue in society at large about the general social conditions and the choice of additional decision-making parameters. The decision as to which priority value for money is given in such decision-making processes is in the end a political one. Here it

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must be borne in mind that political decision-making processes are also influenced by other aspects which in some cases compete with the aim of maximising the benefit for society. This refers in particular to just and fair allocation, political motives aimed at accommodating conflicting social interests, together with personal preferences of the corresponding individuals responsible for taking these decisions. At the moment, health economy aspects play a subordinate role in this context [21,22] even though their significance has increased in recent years, particularly in the medication sector.

Finally, regulatory intervention on the medication market is seen to have an effect on society at large. For example, certain effects of controlling and regulatory measures on the medication sector which are desirable from a sociopolitical point of view also have industrial and job-related consequences. Consideration of the value for money of medication in decisions relating to approving and refunding such medication is therefore frequently seen as an impediment to further innovation, in turn having an effect on the international competitiveness of the pharmaceutical companies.

Value for Money as Controlling and Regulation Element for Medication

The degree to which regulatory intervention in the supply, provision and refunding of medical services can be said to be rational is justified in terms of welfare theory. Given the limited financial and human resources, consideration of the value for money in health policy decisions can improve effectiveness and efficiency in the provision of medical services [6]. It must be borne in mind that given the sectoral divisions within the health systems, as a rule corresponding savings cannot be expected to be implemented to the full.

Interventions in the medications market can also be justified by special aspects of the market, resulting primarily from the asymmetrical distribution of information between doctor and patient. As a result of these special aspects, in many cases patients are only able to assess the benefit or necessity of certain drugs and medication to a limited degree if at all, and depend on the advice and decisions of the doctor treating them. This means they are not free in their demand behaviour and, under certain circumstances, would refrain from claiming a medically indicated benefit or lay claim to such a benefit over and beyond the medically indicated need. And anyway, given their more or less comprehensive insurance cover, the patients have little interest in doing so. With full insurance cover, rational patient behaviour is to claim benefits through to saturation point (moral hazard) [21].

From a formal point of view, inclusion of the value for money aspect in decisions on refunding the costs of medication reinforces the demand side. But first and foremost the demand side is to be seen as the affected insurance company; the patients themselves are only affected indirectly through the level of health insurance premiums. As far as the pharmaceutical industry or supply side is concerned, when the health insurance companies include the value for money aspect in decisions on refunding the costs of medication, this increases the business risk, makes the costs for research and development more expensive and prolongs the time it takes to launch their products on the market.

In principle, it is conceivable for the value for money aspect to be included in decisions on both the approval and the refunding of medication. But on an international scale, there are no examples of value for money being used as an approval criterion. As far as the regulations are concerned, an intervention of this nature in the basic rights of pharmaceutical companies requires corresponding legitimation in law [9]. Similarly, this approach would also affect the consumer's basic rights, because an intervention in the consumer's freedom to decide could only be justified in terms of drug and medication safety. From a medical point of view, it can be argued that a refusal to approve of medication would in fact deprive society of the future benefits of such medication. This could be derived from the growth in findings and know-how based on application observation of the corresponding medication. But the same argument also applies in formal terms, although with lesser consequences, to negative decisions in terms of refunding the medication. If the approval procedure were to start considering decision-making parameters which go over and beyond aspects of drug safety, society could be deprived of the benefits of medication which may not have a socially acceptable value for money at the point in time of the approval procedure, but this criterion could become more favourable in time. Such a phenomenon could take place for example through scaling and learning effects both in manufacture and in application, but at the latest after the patent period has expired. Moreover, in methodological terms it can be questioned whether value for money as evaluated in social terms is a suitable measure for the individual frame of reference. The decision on the individual benefit should depend solely on the individual's willingness to pay [7].

Given the dynamic development of both social and scientific processes, the question arises whether society's willingness to pay should be used as a decision-making criterion in the approval of medication, in view of the fluctuations in this aspect and the associated moral concepts.

It must, however, be presumed that the addition of the refund criterion value for money to the established approval criteria quality, safety and effectiveness will have an effect on market access and on the process of market diffusion of medication. This is where the expression „fourth hurdle" comes from as a continuation of the three other approval criteria. The individual willingness to pay for medication, which is taken to be very low, has made a major contribution to arriving at this expression. Yet there is certainly a willingness to pay for medication in spite of extensive insurance cover, when it comes to products outside the defined benefits package, as illustrated by the example of Viagra® and the increasing volume of over-the-counter (OTC) products.

On an international scale, the consideration of value for money affects decisions regarding the refunding suitability of medication and the prices for such products. The controlling principles can be divided into different categories according to their effects on the individual players in the health system [12]:

- *Regulatory control*
  - *Decision on market suitability*
  - *Decision on refunding suitability*
- *Informational control*
  - *Voluntary social learning*
  - *Implicit support of the political decision-making process*

Taking these control principles, countries can be classified into two rough categories. In Australia, Canada, Finland, Holland and Portugal, information about the value for money aspects of medication is an obligatory component in the decision on refunding suitability, whereas in Denmark, Ireland, New Zealand, Norway and Switzerland, such information is optional.

We shall now take a look at what experience is available in considering value for money in the context of regulating the use of medication, with the example NICE (National Institute of Clinical Excellence) in the United Kingdom, as a representative of the informal principle, and Australia, where information about the value for money is an obligatory component in the decision on refunding suitability.

Including value for money as a controlling element, illustrated by the example of NICE and Australia

NICE or the analogous program SIGN (Scottish Intercollegiate Guidelines Network) in Scotland consists of formal recommendations or guidelines which aim to change the behaviour of doctors in the application of medication. Together with value for money, these guidelines also consider other factors such as effects on the quality of life of the patients. The guidelines are intended to improve quality by reducing inefficiency and to reduce the imbalanced distribution in the practice of medical services. The background to this latter aspect is so-called „postcode prescribing", which describes the phenomenon of extreme regional differences in prescription behaviour, particularly for new, expensive medication.

But NICE's critics also see it as an instrument for rationing medical services [24]. This reproach would appear to be plausible, because consistent implementation of the NICE guidelines results either in additional expenditure on the macro level or changed allocation decisions on account of limited resources on the micro level. It must be presumed that in reality, both developments are happening at the same time. For the period between its launch and 2002, NICE resulted in guideline-induced additional expenditure amounting to £575 million [16]. According to the Local Health Authorities, consistent implementation of the NICE guidelines is financially impossible under the current funding framework [1]. Altogether, expenditure on medication in the United Kingdom increased by 12% between May 2001 and May 2002 [11].

These developments show that in the context of the existing health system, the NICE recommendations are only capable of curbing cost expansion in isolated cases. The same also applies to the stipulated aim of reducing the imbalanced distribution of medical services [2, 17]. Another factor to be considered is that the consistent implementation of guidelines cannot result in the desired secondary effect of implied rationing measures elsewhere, because the prescription of the corresponding medication is practically legitimated by the guidelines. This is illustrated by the case of Bupropion for smoking cessation: NICE refers to a corresponding guideline indicating that the NHS is obliged to refund this product so that every patient who wants this therapy is entitled to it [13].

Previous experience shows that the NICE guidelines have not managed to achieve the declared targets completely. But on sifting through and processing the evidence for medical effectiveness, they do provide important impetus for the decision-making process on all levels of the health system. Guidelines can contribute to a general economic gain in welfare, even if the associated expenditure is higher than without the guidelines. The amount of accepted incremental value for money in turn depends on society's willingness to pay for health services. The politicians will have to weight up the anticipated increase in expenditure in terms of budget impact analysis. This can result in conflicting targets for the medical and political objectives. At the same time it will therefore be necessary to instigate a general social discussion about reducing the range of existing services, if individual services fail to offer the required value for money.

The principles of the „fourth hurdle" have been implemented with the greatest consistency in Australia. Prerequisite for the refunding suitability of medication in the out-patient sector is inclusion in the PBS (Pharmaceutical Benefit Scheme), which happens on application from the manufacturer. Since 1995, information on the value for money aspects has become obligatory [14].

The decision on the refunding suitability is then taken in three stages [4]:

1. *Basic recommendation for inclusion in the PBS by the Pharmaceutical Benefits Advisory Committee (PBAC)*
2. *Definition of negotiation prices by the Pharmaceutical Benefits Pricing Authority (PBPA) and price negotiations with the manufacturers*
3. *Decision for inclusion in the PBS by the Minister for Health*

There are two different procedures, depending on whether the manufacturer aims to obtain a premium price category for his product during the price negotiations. In this case, the decision on refunding suitability is taken as part of a Major Submission, in which information about the value for money is mandatory. If the corresponding medication is a generic drug so that the manufacturer is not aiming for a premium price, information on the value for money is not necessary as part of a Minor Submission. The information on value for money accepts cost minimising, cost effectiveness and cost benefit value studies, and the pharmaco-economic models can also be used.

An appraisal of the effects of the described regulatory measures in Australia reveals that here too, the expenditure for medication has increased sharply in relation to the other sectors, with Australia witnessing an escalation rate of 13.8% for the fiscal year 2001–2002 compared to the previous year [4]. This development shows that in Australia, the „fourth hurdle" cannot be said to have had the hoped effect for controlling to the full. Together with demographic reasons, medical progress is also cited as a reason here, above all incorrect use of medication in the terms of the approval. This refers to symptoms which could be treated just as effectively with less expensive medication. This

phenomenon is referred to as leakage, and cannot be given adequate consideration in estimations of the future market significance. For example, during the approval procedure for Celebrex®, it was presumed that the drug would account for turnover of AUS$ 40 million in the first year after approval. But in fact the turnover in the first year amounted to AUS$ 160 million. Similarly, prior to approval the number of potential patients for treatment of reflux oesophagitis with proton pump inhibitors was estimated at 35,000 p.a. But in the first year following market launch, 177,000 patients were treated with this group of substances [4].

Outlook

In spite of the described inadequate controlling properties in the sense of curbing expenditure, it can be presumed that data on the value for money of medical services in general and medication in particular can make an important contribution to more rational decision-making processes. For example, such data can result in new medication being used more efficiently, for example by identifying sub-groups of patients who could profit in particular from the new drug. Similarly, guidelines as part of disease management measures can also help to overcome sectoral barriers.

Regardless of the institutional conditions for considering the value for money of medication as controlling parameters, an inter-sector view of the cost effects of medication therapies is necessary because this is the only approach that illustrates all relevant costs. In addition, division of the individual service and benefit areas into separate sectors can prevent the use of cost-effective medication which is more expensive than the corresponding therapeutic alternatives. Together with this application hindrance, the volume of savings actually made in these general conditions is less than optimum.

It must also be presumed that inclusion of the value for money as a controlling and regulatory parameter for medication will influence the speed with which innovations are able to diffuse the medication market. Possible delays in approval procedures could have general economic drawbacks (including poorer quality of life for patients and increased mortality rates). From the manufacturers' point of view, such a procedure would reduce the period of exclusive marketing rights during the patent protection period and thus increase the corporate risk. Accordingly, the period for deciding on adequate value for money as a prerequisite for refunding by the health insurance funds and companies must therefore be limited to a maximum period. Another conceivable possibility is to implement a „fourth hurdle" within a fixed period after approval. In this case, medication can initially be included in the list of refunded products but then have to prove its value for money within a five year period, for example, or lose its qualification as refunded product.

It is a conceivable for the value for money aspect to be considered both in the approval criteria and in the context of the refunding suitability of medication. The former would appear to be highly improbable, among other things in terms of competition law. The inclusion of value for money as an explicit rationing measure in the decision on the refunding suitability of medication requires a corresponding statutory basis with major changes to legislation. This process affects numerous aspects, including for example socially just and fair distribution, together with possible interference with individual rights, so that any such changes can only be expected in the medium to long-term at the most.

On the basis of current findings, a „fourth hurdle" can scarcely be expected to curb expenditure. In this context it must also be borne in mind that the structural and organisational requirements to implement a „fourth hurdle" in turn entail additional costs. The decision for increased consideration of the value for money of medication must therefore itself be appraised in terms of its own value for money. In addition, each introduced set of regulations changes the behaviour of those directly effected. This in turn could result in substitution effects which in the end would cause higher costs [20].

For the pharmaceutical industry, the increased priority of the value for money of medication in the context of refunding suitability is associated with changes on the R&D front. This has already resulted in reorganisational measures in favour of pharmaco-economic departments, which are expected to become increasingly important in strategic and operative decision-making processes [5].

Pharmaco-economic aspects affect both clinical phase II and Phase III of clinical research during product development. Whereas in phase II pharmaco-economic simulations can support decisions for later development phases, the specific generation of pharmaco-economic data is advisable in clinical phase III. This approach is capable at least of partly avoiding future retrospective model calculations.

Pharmaco-economic simulations in the clinical phase II of medication development can, for example, identify patient sub-groups with a relatively more favourable value for money compared to the potential total cohort. Such risk groups can then be given special consideration during the planning for the clinical phase III. This procedure is advisable particularly in view of the NICE recommendations, which as a rule refer to the evaluation of the corresponding mediation for special sub-groups and not for a total patient cohort. Similarly, such an approach can also help in choosing the effect parameters for the clinical phase III. As well as contributing to the operative decisions during clinical research, strategic assistance can also be provided in the sense of „Go/No-Go" decisions for new substances, which can influence the product portfolio of a company [10].

By contrast, clinical phase III should already consider pharmaco-economic data while planning the study design [15]. This approach would also appear advisable in view of the fact that as a rule, HTA programmes explicitly address the workforce of pharmaceutical manufacturers.

To conclude, a consideration of the value for money of medication in the sense of a „fourth hurdle" has consequences in medical, socio-economic and also industrial terms. In view of the fact that the implementation of such regulations would affect different, in some case opposing interests, the decision in favour of a „fourth hurdle" for medication will have to be taken as part of the political decision-making process with the participation of all those affected and all social groups.

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