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A comparative study of the role of disease severity in drug reimbursement decision making in four European countries

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ABSTRACT

Considerations beyond cost-effectiveness are important in reimbursement decision making. We assessed the importance of disease severity in drug reimbursement decision making in Belgium, France, The Netherlands and Sweden. We investigated scientific literature and policy documents and conducted three interviews in each country (four in The Netherlands) with persons involved in drug reimbursement. Disease severity is an important consideration, especially where the level is high. The Netherlands operationalizes disease severity using the proportional shortfall approach. Sweden uses categories to give an indication of the level of severity. In The Netherlands and Sweden, severity only implicitly plays a role in the decision whether to reimburse a drug, whereas in Belgium and France it also explicitly plays a role in determining the willingness to use public resources. Interviewees acknowledged that as well as a qualitative description of the disease, quantitative information may also be useful as input for decision making. None of them, however, considered this to be of decisive importance. Although disease severity is important in drug reimbursement decision making in all four countries, all seem to struggle in explicitly specifying its actual role. Belgium and France are the most explicit by using levels of severity in setting reimbursement levels; all four countries could, however, improve the transparency of its actual importance relative to the other criteria in the decision-making process.

1. Introduction

Considerations beyond cost-effectiveness and safety are important in reimbursement decision making [1–6]. One such consideration is disease severity or any other operationalization of 'medical need'. Velasco-Garrido et al. [7] found the 'need' aspect to be one of the most considered criteria in nine European countries. Similarly, other studies reported that the more severe a disease, the more valuable a treatment [8–10]; this reflects the equity objective of most health care systems. On the other hand, healthcare interventions may not be reimbursed for diseases of low levels of severity as it indicates a limited 'need'. Many stakeholders and analysts agree on the fact that the level of severity may play a role in reimbursement decision making. However, the way disease severity contributes to the decision-making process is often not transparent and may also differ between countries [4,11].

Previous studies investigated the importance of considerations beyond cost-effectiveness, for example, by

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eliciting policymakers' preferences [12,13], or by eliciting public preferences regarding the social value of the quality adjusted life year (QALY) [14–16], preferences for severity of illness [10,17,18], for end-of-life treatment [19,20], or for the rule-of-rescue [21]. These studies provided insights into the (relative) importance of the severity of the disease. There is still a lack of clarity, however, regarding the implementation in actual practice. A comparison across countries on the role of disease severity in reimbursement decision making may provide important insights into actual policymaking. It may, therefore, provide guidance to policymakers on how to incorporate this criterion in health care resource allocation as many countries seem to experience difficulties in doing so.

We investigated the role of disease severity and its operationalization in drug reimbursement decision making in four European countries: Belgium, France, The Netherlands and Sweden. We explored concepts of disease severity and assessed how information on disease severity is used in everyday drug reimbursement decision making. This paper addresses the following questions: (i) is disease severity considered in drug reimbursement decision making; (ii) if considered, how or by which method or indicator is it presented; and (iii) in what way is this information used in practice and how does it affect decision making.

2. Context

All four countries have a national drug reimbursement agency. Within the agency, a technical department prepares the assessment and preliminary reports. An independent pharmaceutical expert advisory committee assesses and appraises the evidence and is responsible for advising the final decision maker (i.e., the minister of health). Notably, the ministers hardly ever deviate from the advice [5]. In Sweden, the expert advisory committee also makes the final decision. Only The Netherlands has a separate appraisal committee which has an advisory function. All four countries use drug effectiveness and cost-effectiveness as reimbursement criteria. In France the use of cost-effectiveness as a criterion has only recently been introduced. This paper focuses on the role of disease severity in decision making. For more detailed information on the four systems regarding their (other) reimbursement criteria, reimbursement process, and their health technology assessment (HTA) models, we refer to Franken et al. [5] and le Polain et al. [11]. Table 1 provides an overview of the four drug reimbursement systems.

3. Concepts of disease severity

Need principles are commonly discussed in academic literature concerning health care rationing decisions [22]. A drug is valuable when it fills a specific need; this may be a medical, therapeutic, or societal need [23]. These needs depend on factors such as treatment necessity and disease severity (medical need), the availability and the effectiveness of alternative treatments (therapeutic need), and the prevalence of the disease and inequalities in health (societal need) [11]. As such, disease severity is part of the 'need principle'; the more severe a disease, the higher the (medical) need.

A large number of empirical studies acknowledge the concept of 'severity' as a prioritising principle [10]. Several approaches to determine 'who are the worst off' are described in the literature to operationalize the concept of severity. According to the '*fair innings*' approach, everyone is entitled to some 'normal' span of health achievement [24]. This implies that treatments for patients who have not yet had their fair innings are valued higher than treatments for patients who have had their fair share. The second group are 'living on borrowed time' according to Williams [24]. This approach considers life time health achievement including the quality as well as the length of a life, thus also including past health (losses). The '*severity-of-illness*' approach prioritises persons with the worse off initial condition based on the severity of the initial health state as well as the expected (prospective) health should no treatment be available [25,26]. This approach does not consider past health. In contrast to the previous two 'absolute' approaches, the '*proportional shortfall*' approach, considers the worse off in relative terms. This approach bases the need on the proportion of health lost due to the disease as compared to the expected health (i.e., level of health and remaining life expectancy) without the disease [27]. Finally, *the rule of rescue'* approach prioritises identifiable individuals facing avoidable death, regardless of the costs [28]. It should be noted that, if used for priority setting, all approaches can identify a group of people (or an individual) who are the worst off. The last approach, however, deviates because it concerns '*identifiable*' individuals who are worst off irrespective of the medical diagnosis (e.g. identifiable by television or media), whereas the others concern '*non-identifiable'* groups of individuals per disease and concern a measure of loss. Therefore, only the first three approaches facilitate a numerical expression of the levels of severity of a disease that could be used in reimbursement decision making at the national level.

4. Methods

We compared the role of disease severity and its operationalization in drug reimbursement decision making in Belgium, France, The Netherlands and Sweden. The selection of these countries was based on our previous research on European drug reimbursement systems [4,5,11,29]. All four countries have established HTA agencies using reasonably comparable reimbursement processes.

To obtain insight into the role of disease severity in actual decision making, we first evaluated the reimbursement processes and criteria. Second, we explored scientific literature describing concepts of disease severity. Third, we conducted face-to-face interviews; three in each country (four in The Netherlands because of the existence of the appraisal committee). Fourth, using data triangulation we combined the information from the literature, policy documents and the interviews to assess the role of disease severity and its operationalization in drug reimbursement decision making.