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A multi-criteria decision approach for ranking unmet needs in healthcare

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ABSTRACT

Early temporary reimbursement (ETR) schemes for new interventions targeting high unmet needs are increasingly applied in pharmaceutical policy. Crucial for these schemes is the assessment of unmet healthcare needs of patients and society.

This study develops and tests a multi-criteria decision approach (MCDA) for assessing therapeutic and societal needs. The Belgian unmet needs commission, responsible for creating a list of unmet needs for the ETR programme, has tested this methodology to assess the needs in eight health conditions. For therapeutic need, three criteria were included (impact of the condition on quality of life and on life expectancy and inconvenience of current treatment); for societal need two criteria (condition-related healthcare expenditures per patient, prevalence).

The results show that the proposed MCDA is feasible and acceptable for the unmet needs commission. Clear definitions of the criteria and regular repetition of these is needed to avoid variable interpretation of the criteria by the commission members. Quality assessment of the evidence is desired. Rankings resulting from the application have face validity. Considering therapeutic need separately from societal need is considered appropriate.

Policy makers should consider the use of MCDA in assessing healthcare needs. MCDA improves the transparency and accountability of the decision making processes and is practical and feasible.

1. Introduction

Access to innovative medicines for high unmet medical needs has been a concern for policy makers since many decades in view of the rapidly evolving discovering by the pharmaceutical industry of personalized treatment options and not the least in view of the escalating costs related to this. On the regulatory side, early access schemes have been developed in the EU, e.g. compassionate use programmes, accelerated assessment, conditional marketing authorization and the PRIME initiative (priority medicines) [1,2]. The objective of these programmes is to give early access to promising treatments for patients suffering from severely debilitating or life-threatening health conditions for which no treatment currently exists.

However, none of these programmes automatically implies reimbursement. Early access is in that case a relative concept, as without reimbursement, patients will most often still not have real access. While governments want to help patients with high unmet medical needs in the best way they can, they also have to guarantee the sustainability of the healthcare system and the equity and efficiency of resource allocation. Therefore, early temporary reimbursement (ETR) schemes have been developed in several countries, implying a temporary financial contribution to the costs of using products with a yet not fully determined or uncertain relative effectiveness, safety profile, budget impact and cost-effectiveness [3–6]. In Belgium, a law was established in 2014, organising the possibility to grant a temporary financial compensation to companies providing their promising products that have not received marketing authorization yet to patients with high unmet medical needs. The decision is taken by the College of Medical Directors (CMD) for a cohort of patients fulfilling certain well defined conditions after advise of the "Commission for advice on temporary reimbursement of a pharmaceutical product" founded within this NIHDI, hereafter called the "unmet needs commission".

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The CMD is a college within the National Institute for Health and Disability Insurance (NIHDI) responsible for assessing specific therapeutic needs, needs for innovative interventions and complex care in rare indications and diseases. The unmet needs commission prepares the decisions of the CMD by assessing the level of unmet medical need in the indication and the extent to which the proposed product is eligible for compensation under the unmet needs programme (for the NIHDI organisation chart: http://www.riziv.fgov.be).

The initiative to submit a request for early access and ETR can come from a company, the CMD itself or the minister of public health and social affairs. The financial compensation consists of two components: a fixed amount of 20000 Euro per file submitted by a pharmaceutical company (covering part of their administration costs) and an additional amount of 2500 Euro per patient per year, covering the product costs. This amount is identical for every treatment compensated under the unmet needs programme. The compensation does not determine the final list price of the product. The price setting happens afterwards, as part of the regular reimbursement procedure after EMA approval.

To be able to judge whether a product is eligible for ETR, a list of unmet medical needs is created each year by the unmet needs commission. Proposals to put a disease or condition on the list of unmet needs can be submitted by companies, the minister of public health and/or social affairs and the college of medical directors. The proposals are appraised by the unmet needs commission and put in a rank ordered list. The appraisal of the medical need in a particular disease involves the consideration of multiple criteria. It is challenging to balance these different criteria in a consistent manner across diseases.

The objective of this study was to develop a multi-criteria appraisal methodology for ranking the unmet needs of diverse patient populations, to pilot-test this methodology in the unmet needs commission and to assess its acceptance by this commission.

2. Methods

2.1. Multi-criteria decision analysis

A multi-criteria decision analysis (MCDA) approach was chosen. MCDA can help decision-makers to structure complex decisions that involve multiple criteria. MCDA is a generic term that encompasses several methods and approaches, each with the common goal to make explicit the impact of criteria on the decision made and their relative importance [7].

MCDA encompasses several steps. For each step methodological choices had to be made. The procedure followed in our initiative is presented in Fig. 1.

2.1.1. Step 1: definition of the decision problem and validation with decision makers

The decision problem was defined as "making a judgment on the relative healthcare needs in diverse diseases". This definition was based on the request of the unmet needs commission submitted to the Belgian Health Care Knowledge Centre (KCE), the Belgian HTA agency, to develop a methodology for this purpose.

2.1.2. Step 2: identification of relevant decision criteria

The criteria identified in a previous Belgian study about public preferences for criteria to assess therapeutic and societal needs were used [8]. The study identified 3 criteria for assessing therapeutic needs and 2 criteria for assessing societal need (Table 1). These criteria were selected from a longlist of criteria by an expert panel consisting of multiple stakeholders, making sure that the retained criteria, as defined, satisfied the technical requirements for MCDA, i.e. being non-redundant, non-overlapping, independent [9]. Preference dependence was not considered in our MCDA. This was not possible within the MCDA approach that we applied. With other approaches, like the analyses of responses to discrete choice experiments, interaction terms could be included in the model, but as interaction terms by definition overlap with other included criteria, we did not include them in our MCDA model.

Table 1
Criteria for defining therapeutic need and societal need and their weights.

Therapeutic need	**Weight***
• Impact of the condition on quality of life with current treatment.	0.43
• Impact of the condition on life expectancy with current treatment.	0.14
• Inconvenience of current treatment.	0.43
Societal need	**Weight***
• Condition-related public expenditures per patient.	0.65
• Frequency of the condition (prevalence or incidence).	0.35

Source: Cleemput et al. [8].
* weights are expressed on a 0–1 scale. The higher the weight, the more important the criterion.

The distinction between therapeutic need and societal need as defined in the previous study is maintained as, according to the decision makers, it is intuitive and relevant. "Therapeutic need" refers to the need for a better treatment than the treatment currently reimbursed, from the perspective of the patient [10]. It can refer to the need for a treatment because none is currently available, but also to the unresolved issues in an existing treatment for a specific disease. Therapeutic needs are needs as perceived by individual patients and are independent from the needs of the society. Because of the important difference in perspective (societal versus individual), decision makers consider difficult to weight societal needs against therapeutic needs in one system [10]. It would entail the question of whether the individual patient needs are more important or the societal needs. For policy makers this is an awkward trade-off, as societal needs are of a different order than patient needs. Budgetary concerns, for instance, are only relevant because there are opportunity costs (you can only spend resources once). Opportunity costs relate to other patients. They are irrelevant for judging the severity of a disease or a specific patient population's needs. The current Belgian legislation states that conditions can come on the unmet needs list if it is a seriously debilitating or life-threatening condition for which no appropriate reimbursed therapy is available. The "appropriateness" of a therapy could be interpreted as its effectiveness, and a condition could be interpreted as any symptom of a disease that is not under control [10].

Impact of the condition on quality of life with current treatment refers to the extent to which the disease has an impact on the five dimensions of the EQ-5D, a generic health-related quality of life instrument frequently used in research: mobility, self-care, usual activities, pain/discomfort, anxiety/depression. Mobility refers to the ability to walk about, self-care to the extent to which patients are able to wash and dress self, usual activities to the extent to which they are able to participate in social activities and go to work or school.

Impact of the condition on life expectancy refers to the extent to the number of years people lose due to their disease despite current treatment, as compared with patients of the same age without the disease.

Inconvenience of current treatment can refer to inconvenience caused by, for instance, the frequency of use (e.g. taking a drug once or more times a day), the administration route (e.g. syringes, oral drugs, via perfusion, by the patient him- or herself or by someone else), the place of administration (in hospital, at home, in a

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