BRIEF REPORTS

A Pilot Study of Multicriteria Decision Analysis for Valuing Orphan Medicines

Jon Sussex, MSc1*, Pierrick Rollet, MBA2, Martina Garau, MSc1, Claude Schmitt, MSc1, Alastair Kent, MPhil4, Adam Hutchings, MSc5


ABSTRACT

Objective: To pilot the use of multicriteria decision analysis to establish and apply a framework of weighted attributes to value orphan medicinal products. Methods: Literature searches on the natural history and burden of 40 rare diseases and of how payers assess treatment value and three workshops with, respectively, GlaxoSmithKline managers working on orphan medicinal products, European Union clinical and health economics experts, and representatives of rare diseases patient groups in the European Union. Results: Eight nonmonetary attributes were identified and weights agreed: four concern the disease being treated and four the treatment itself. About half of the weight went to attributes of the disease treated and half to attributes of the treatment. Patient group representatives gave greater weight than did the experts to patients’ and carers’ quality of daily life. Conclusions: The multicriteria decision analysis approach piloted works and could be developed for use by payers and health technology assessment bodies. Keywords: HTA bodies, methods, orphan drugs, payers, rare diseases.

Introduction

This article presents an experimental pilot study that tests a multicriteria decision analysis (MCDA) approach [1] to establish a framework for valuing orphan medicinal products (OMPs) and providing an explicit understanding of trade-offs for decisions on their eligibility for funding.

All health care systems’ health technology assessment (HTA) and reimbursement decisions depend on an implicit, if not explicit, assessment of value as the first step. Efforts by policymakers and payers to better determine the value of medicines are widespread internationally. The 2011 AMNOC (Arzneimittelmarktneuordnungsgesetz, or medicines market restructuring law) reforms in Germany and the development of “value-based pricing” in the United Kingdom are two high-profile examples [2,3] among many others [4,5]. No HTA agency yet uses MCDA, but the European Medicines Agency is developing an MCDA approach to balancing the benefits and risks of new medicines considered for licensing [6] and National Health Service England has proposed what is in effect an MCDA process for deciding which oncology medicines will be funded by the national Cancer Drugs Fund for National Health Service patients in England [7]. The literature on MCDA in health care is growing [1,8].

MCDA is a set of methods to aid decision making where more than one criterion is relevant, which make explicit the impact on the decision of all the criteria and the relative importance attached to them. The main steps are (see [1] for more detail) as follows:

- establish the decision context—what is to be decided, by whom;
- identify attributes for assessing the value of each medicine;
- assign weights to the attributes to indicate their relative importance to the decision;
- score the expected performance of each medicine against the attributes;
- combine weights and scores to indicate overall value; and
- consider the implications of the results and test their sensitivity to reasonable variations in weights and scores.

Variants of MCDA range from those using sophisticated algorithms to identify the total (dis-)benefits of an option to more basic approaches limited to providing and recording a structured and explicit deliberative process. All forms of MCDA aim to achieve replicability and transparency, and hence accountability, in decision making. MCDA has been extensively used in health care and other sectors (transport, social services, immigration policy, etc.). MCDA aids and structures the exercise of judgment by decision makers but does not do away with the need for that judgment [8].

OMPs are treatments for patients with rare diseases, defined in Europe as conditions affecting fewer than 1 in 2000 people. Rare diseases are often chronic, progressive, and life threatening;
many of them affect children; and there is often a lack of effective treatments for these diseases. Small populations, substantial heterogeneity, lack of knowledge about natural history, and difficulty in defining practical clinical end points create greater uncertainty around evidence in rare diseases than in common ones. The development of OMPs is often accompanied by partial knowledge of diseases and scarce medical expertise. Legislation has accordingly been introduced in the United States and the European Union (EU), establishing special incentives for the development of treatments for rare diseases, and increased numbers of orphan drug designations have followed [4].

Payers commonly treat OMPs distinctly from other medicines. A number of HTA systems have special arrangements for the assessment or reimbursement of OMPs. In England and Wales, treatments for very rare conditions are assessed and commissioned in a separate process from other treatments (until April 2013 by the Advisory Group for National Specialised Services [9] and since then by the National Institute for Health and Care Excellence [NICE]) whose Highly Specialised Technologies Evaluation Committee is building on the work done by the Advisory Group for National Specialised Services [10]. The process uses criteria in addition to health gains, including attributes related to societal value and impact on clinical practice. In Scotland, a special fund specifically for OMPs was set up in early 2013 [11]. At the European level, policy initiatives are aimed at improving the approach to assessing the value of new OMPs. For example, the EUCERD (European Union Committee of Experts on Rare Diseases) [12] is developing processes to inform decision makers about the clinical added value of OMPs and facilitate timely reimbursement.

Winquist et al. [13] have proposed a process for reviewing OMPs by payers that works around problems with demonstrating clinical effectiveness. But we have not been able to find in the literature a value framework for assessing OMPs that sets clinical effectiveness alongside other attributes of value.

Launching a treatment for a hitherto untreated rare disease puts that disease on the clinical map. Clinicians are then more likely to be aware of the disease, to recognize cases that present to them, and to have the necessary skills to help [14]. This suggests that the existence of an unmet need for treatment might be more important when determining the value of an OMP than when evaluating treatments of more prevalent conditions.

For all these reasons, it is important to relate the “significant benefit” value criterion required for OMP designation with a measurement model that can help value judgments. An MCDA process and the development of a comprehensive literature review was impractical. A subset of 40 diseases was selected on the basis of availability of literature on morbidity, mortality, broader patient and carer burden, disease frequency, severity, degree of scientific understanding, and progress in developing effective treatments. Searches were conducted in MEDLINE, EMBASE, the Cochrane database, Orphanet, and the EURORDIS patient association Web site. For each condition, disease impact was broken down by individual or group affected (patients, family, society), nature of the effect (pathological, clinical, symptomatic, outcomes, economic), and the proximity of the effect to the primary manifestation of the disease.

A second search looked for how existing payer frameworks estimate treatment value in 10 OECD (Organisation for Economic Co-operation and Development) countries with OMP regulatory pathways and well-established pharmaceutical reimbursement processes (Australia, France, Germany, Italy, Japan, The Netherlands, Spain, Sweden, the United Kingdom, and the United States). A related search focused on rationales given in reimbursement decisions for OMPs in those EU countries where the reports were available in English: the United Kingdom (NICE and SMC [the Scottish Medicines Consortium]), France (Transparency Commission), and Germany (GBA [Gemeinsame Bundesausschuss, or the Joint Federal Committee—IQWiG [Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen, or the Institute for Quality and Efficiency in the Health Service]). These searches were supplemented through 10 interviews with clinical experts, academics specialized in health economics and policy, and rare diseases patient group representatives in the EU and the United States.

This process yielded 14 attributes. Practical guides to MCDA recommend using fewer than 10 attributes. We excluded the net monetary cost impacts of the disease and the treatment, as to include them would require monetary values for all the non-monetary attributes. We sought instead to establish the value of an OMP to set against its net cost impact. We discussed the attributes at a workshop in March 2012 with GSK managers working on the development and commercialization of OMPs, and aggregated them into the following eight attributes:

- **impact of the rare disease and associated unmet need:**
  1. availability of effective treatment options/best supportive care in the absence of the new medicine;
  2. disease survival prognosis with current standard of care;
  3. disease morbidity and patient clinical disability with current standard of care;
  4. social Impact of the disease on patients’ and carers’ daily lives with current standard of care;

- **impact of the new medicine:**
  5. treatment innovation, defined as the scientific advance of the new treatment together with contribution to patient outcome;
  6. evidence of treatment clinical efficacy and patient clinical outcome;
  7. treatment safety; and
  8. social impact of the treatment on patients’ and carers’ daily lives.

The rationales for these attributes and their particular relevance in rare diseases, with references to the literature from which they are drawn, are detailed in Supplemental Materials found at http://dx.doi.org/10.1016/j.jval.2013.10.002. The inclusion of attributes of the disease, as well as of the treatment itself, is recognized as relevant by various authorities (e.g., [3] and [17]).

To provide a combined value assessment based on these attributes, we used an MCDA approach. We selected a “value measurement model” [8] as being of most value to HTA and

**Methods**

We identified an initial list of value attributes from a literature review of rare diseases, a review of HTA for OMPs, and interviews with clinical experts, economists, and representatives from rare disease patient groups. A literature search was undertaken on the natural history and burden of 40 rare diseases (see Appendix A in Supplemental Materials found at http://dx.doi.org/10.1016/j.jval.2013.10.002. There are more than 7000 rare diseases, and so a
reimbursement decision makers. Weighting of the eight value attributes and using them to rate example OMPs were first piloted in the March 2012 workshop with GSK managers and then formed the basis of two further workshops:

- with clinical and health economics experts from France, Germany, Italy, Spain, and the United Kingdom in April 2012; and
- with representatives of rare diseases patient groups in the EU in August 2012.

Each of these 1-day workshops included 6 to 11 participants plus the authors of this article as facilitators. In addition, medical/scientific specialists were on hand to provide factual information and clarification about the OMPs assessed. The workshops were highly structured and included participants working in small subgroups (three to four per subgroup) to complete tasks within strict time limits.

Following an introduction explaining the purpose and nature of the workshop, the first substantive session in each workshop was devoted to validating the set of value attributes. Participants at the April and August workshops were offered the opportunity to change the list of attributes agreed at the March workshop if they had concerns, but they were content with the eight attributes.

We took a societal perspective when establishing the value attributes, while recognizing that payers and HTA bodies in some countries currently take narrower perspectives limited to clinical effectiveness or health gain [18]. We asked workshop participants when determining attributes’ relative weights to take into account the interests of all relevant stakeholders including patients, their families and carers, payers, and the national economy.

In the second session of each workshop, the participants assigned relative weights to the attributes. Participants were divided into groups of three to four plus a facilitator. Before breaking into the groups, participants were asked to consider by themselves all the attributes and to allocate each of them initially to one of three headings (“high,” “medium,” or “low” importance) for determining the value of an OMP. Participants then discussed in their small groups how to allocate 100 weighting points across the eight attributes. Each group reached a consensus weight out of 100 for each criterion. The individual groups’ weightings were then reported to a plenary session, any significant differences between groups’ weightings were discussed, and each group was given the opportunity to revise its weightings. It always proved possible in the plenary discussion to reach a consensus weighting for each attribute: all participants were content to accept an average of the groups’ weightings, as amended following the plenary discussion, where there remained any difference in those weightings.

An important part of the study was to test the views of rare disease patient groups, clinical experts, and health economists about the balance of weights between the two groups of attributes: those related to the disease being treated and those concerning the effectiveness of the new medicine. Empirical studies support the use of the severity of the disease being tackled as a criterion for determining the value of a treatment although the exact strength of that support is less clear [19]. There is less evidence about the importance of unmet need per se although what there is does suggest that it is relevant [20].

After establishing the attributes’ weights, the workshop participants rated two case study OMPs from the GSK pipeline for each of the eight attributes and using them to rate example OMPs were first piloted in the March 2012 workshop with GSK managers for the project, sensitivity analyses were conducted in front of the workshop participants, on the basis of combinations of adjustments to relative weightings of criteria and to scorings against each criterion, within the ranges of weights and values that had been discussed in the earlier plenary sessions.

### Results

We report the outcomes of the two workshops with participants invited by, but external to, GSK, namely, a group of European clinical and health economics experts at the April workshop and a group of European rare disease patient group representatives at the August workshop. The results are presented as a “proof of concept” rather than as definitive values.

Both workshops proved successful as pilots of the MCDA process. All participants proved able and willing to engage with the tasks in all sessions of the workshop, and a consensus was agreed with respect to attribute weightings and to the scoring of medicines against those attributes.

Table 1 - Profiles of the two OMPs rated in the MCDA workshops.

<table>
<thead>
<tr>
<th>Value attribute</th>
<th>Treatment A</th>
<th>Treatment B</th>
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<tr>
<td>Availability of effective existing treatment options</td>
<td>Yes</td>
<td>No</td>
</tr>
<tr>
<td>Main target patient outcome</td>
<td>Survival</td>
<td>Progression of disease</td>
</tr>
<tr>
<td>Therapeutic area</td>
<td>Immunodeficiency 250–600 patients</td>
<td>Neuromuscular 3000–8000 patients</td>
</tr>
<tr>
<td>Prevalent population range in the European Union</td>
<td>Open clinical trial (no control group)</td>
<td>Randomized double-blind placebo controlled</td>
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<td>Pivotal trial—data package</td>
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MCDA, multicriteria decision analysis; OMPs, orphan medicinal products.
weight to whether development of the medicine brought a scientific advance, with the stated rationale that when the other seven attributes are taken into account, this attribute would not be seen by patients, care takers, or health care payers as adding any further value.

The rare disease patient group representatives differed from the clinical and health economic experts in some aspects of the weightings (Table 2). The patient group representatives spread the weights more equally across the eight value attributes. They gave more weight than did the clinicians/economists to the impacts of the disease, and of the new treatment, on individual patients’ and carers’ daily lives and were also willing to give some weight (5%) to treatment innovation/scientific advance. The (un-)availability of existing treatments was less important to the patient representatives than it was to the clinicians/economists. So too was evidence of treatment clinical efficacy and patient clinical outcome, although this remained the (equal) most important criterion, as it had been for the clinicians/economists.

Overall, both sets of workshop participants agreed, independently, to give slightly more weight to the attributes of the disease being targeted than to the impacts of the new medicine aimed at it: around 53% versus 47%. This result was discussed in the plenary session at each workshop and each time was confirmed as the collectively desired balance.

How the two case study treatments were rated against each of the eight benefits attributes at first the experts workshop and later the patient groups workshop is shown in Tables 3 and 4, respectively. At each workshop, the participants found it easy to agree after discussion a consensus rating for each attribute and treatment. The clinical and health economics experts rated Treatment A highly: a total weighted score of 580.5 (within a

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<tr>
<td>Availability of existing treatments</td>
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<td>Disease morbidity and patient clinical disability with current soc</td>
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<tr>
<td>Social impact of disease on patients’ and carers’ daily lives with current soc</td>
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<td><strong>Subtotal weight for impact of disease/extent of unmet need</strong></td>
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<td>Treatment innovation: scientific advance + contribution to patient outcome</td>
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<tr>
<td>Evidence of treatment clinical efficacy and patient clinical outcome</td>
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<td>Treatment safety</td>
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<td>Social impact of treatment on patients’ and carers’ daily lives</td>
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<td><strong>Subtotal weight for impact of new medicine</strong></td>
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<td><strong>Total</strong></td>
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soc, current standard of care.

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<th>Table 3 – Ratings of the OMPs from experts workshop.</th>
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OMPs, orphan medicinal products.

<table>
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<th>Table 4 – Ratings of the OMPs from patients workshop.</th>
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OMPs, orphan medicinal products.
possible range of 100–700, with 700 being the best possible). They rated Treatment B somewhat lower overall: a total weighted score of 538.5. The minimum individual rating they gave for any attribute for either treatment was a “4” for Treatment B for three of the treatment attributes, and the highest individual rating they gave was “7” (out of 7) for two of the disease characteristics of Treatment B and for one disease characteristic and one treatment characteristic of Treatment A (Table 3).

Table 4 shows that the patient group representatives, using their weights for the attributes and their assessment of the scores for the case study treatments against those attributes, rated both treatments more highly than did clinical and health economics experts, but like the experts they rated Treatment A above Treatment B. Their reasons for rating Treatment A above Treatment B overall were broadly the same as the experts’: better treatment outcomes achieved by Treatment A. Where the two sets of workshop participants differed from one another most was in their ratings of the diseases being treated. The experts considered disease B to be untreated currently (rating = 7 for “availability of existing treatments”) but that the current standard of care for disease A did provide a little relief for patients (rating = 5). The patient group representatives considered both diseases to be equally poorly treated currently (rating = 6 for both). Conversely, the experts saw the survival prognosis as equally poor for disease A and disease B (both rated at 6), whereas the patient group representatives saw the prognosis as being rather worse for sufferers from disease A (rating = 7) than for sufferers from disease B (rating = 5).

The process summarized in Tables 3 and 4 made explicit the trade-offs made between different value attributes in reaching the overall assessment of value for each OMP. For each attribute, workshop participants were able to discriminate between factors they thought did or did not impact value of treatment. Sensitivity analysis showed that only with implausibly large changes to attributes’ weights would the ranking of the two case study medicines by either workshop change. For example, using the patient groups’ attribute weights and experts’ ratings, the overall weighted scores for Treatment A and Treatment B would become 601.5 and 541, respectively. Using experts’ weightings and patient groups’ ratings would give overall scores of 634 and 551.5, respectively, for Treatment A and Treatment B.

Discussion

We discuss the following:

- how our findings compare with other literature on MCDA in HTA;
- the appropriateness of our MCDA approach compared with alternatives;
- the practicality of an MCDA-based approach to aid real-world decision making by payers and the HTA bodies that advise them; and
- future work to build on the pilot study.

The principal motivation for our pilot study is the absence from the literature of explicit, weighted, value frameworks for OMPs. Most use of MCDA in health care has been by health care payers attempting to prioritize options at a relatively high level; a concise survey of such MCDA studies is given in Devlin and Sussex [1]. We are not aware that any HTA or P&R body currently states that it applies MCDA methods. However, some HTA bodies measure health gain as QALYs, and we note that the process of determining the dimensions of health state that are included (e.g., in the EuroQol five-dimensional questionnaire or other instrument) and then of weighting those health states relative to one another via value sets is itself an MCDA process. It is but one logical step further to consider a set of attributes beyond QALYs alone, and derivation of weights for those attributes, for use in HTA. We have focused on OMPs, noting that HTA and payer bodies often assess them via processes that are distinct from those for other medicines because of the special issues OMPs raise (see the Introduction).

One of the most striking findings from our pilot study was the large weight given to the nature of the disease being treated, as distinct from the result of using the medicine to treat it. There is a growing literature on the role of disease severity and of the hitherto unmet need in valuing health care interventions [19,20], which is consistent with our finding, which was common both to the patient group representatives and to the clinical/economics experts.

Our pilot found that the patient group representatives gave greater weight than did the clinical/economics experts to attributes concerned with the quality of the day-to-day lives of patients and their carers. This makes intuitive sense—the patient group representatives are more aware of the practical implications of living with rare conditions—but we are not aware of other studies comparing the attribute weights of patient groups versus clinical/technical experts and so do not know whether this kind of difference extends to less rare conditions or other MCDA processes.

Identification of the best form of MCDA for payers and their HTA bodies to value OMPs remains an empirical question. There is a continuum of approaches ranging from algorithmic methods to more deliberative processes that allow for exceptions to be made [1]. Among the more algorithmic approaches there are various ways of identifying attributes and their weights and of comparing options, for which there is no space to go into in this short article (the interested reader is recommended to refer to [1] and [8] for detailed but accessible discussions). Each approach has advantages and disadvantages. Decision makers may be reluctant to submit themselves to public scrutiny, but secrecy damages public confidence in their decisions. MCDA approaches increase the defensibility of decisions. Therefore, on balance we believe that a way forward is where an MCDA tool is used to help decision-makers’ deliberative processes rather than being applied in a mechanistic way.

We identified value attributes via an extensive literature search and a deliberative process at a workshop. Weights were not available from the literature; they were determined via a deliberative process as an integral part of the MCDA itself. Because this was a pilot study, it was important to have the opportunity to discuss in detail with experts and rare disease patient group representatives both the attributes and how they were weighted. This implied a workshop approach rather than a discrete choice experiment (DCE) or use of “remote” tools such as “1000Minds” [1].

We use a “value measurement model” to assess an OMP against the current standard of care and to permit comparison of the value of different OMPs treating different rare conditions. This has the advantage of being easier to use and present to non-technical audiences, relative to “outranking” and “goal programming” approaches (see [8]).

The way we conducted our pilot could be replicated in real-world settings by the decision-making groups with HTA and reimbursement bodies, for example, the Appraisal Committees and Citizens Council employed by NICE in the United Kingdom. Any MCDA process based on workshops of limited scale and duration may be criticized for superficiality, but HTA and P&R committees suffer equally in this regard. Pragmatism dictates the time and resourcing in all cases. None of the participants in our 1-day workshops, several of whom have worked on or advised HTA bodies and their committees, expressed an unwillingness to agree and weight value attributes, and then rate treatments against those attributes.
Weighting attributes and rating how well an OMP achieves each attribute are not particularly difficult tasks for an individual to perform. Potentially more difficult is to ensure that when a number of people’s views are sought, everyone’s understanding of the evidence on a disease and how well a medicine treats it is the same and likewise their understanding of the reason for assessing the value of the medicine. The workshops proved to be an effective way of achieving shared understanding of benefit attributes and buy-in to a collective weighting of them. A similar process would be practical for the appraisal committee of an HTA body.

In countries where there are already separate processes for OMPs, our MCDA tool is evidently capable of being applied by the kinds of clinical and economic experts, and patient representatives, who already sit on and advise (or would like to) HTA bodies. The main barrier appears to be reluctance by HTA bodies and/or payers to be that transparent. But transparency is essential if clear and consistent signals are to be sent to investors in medicines research so that resources are prioritized to those avenues of R&D most likely to produce the OMPs most valued by the people whom health care payers and their agencies exist to serve. A practical and important step in the direction of transparency could be to use MCDA approaches within the deliberative processes of HTA bodies for all their decisions, combined with publication alongside each decision of all the attributes considered and to what extent they did or did not affect the final decision.

To achieve that will require HTA bodies to try MCDA approaches for themselves. This is not a costly undertaking, and it would be reversible if insurmountable obstacles were deemed to have arisen. If a “testing by doing” approach were adopted, value sets and weights would be established de facto and could be refined as MCDA approaches are used repeatedly over time and across decisions. In the long term, it might become possible to conduct a multinational, for example, pan-European, assessment of the value of OMPs (which would leave reimbursement as a country-level decision), in the spirit of the ongoing initiatives in the European policy arena [12].

Development for use in real-world decision-making settings of the value framework we identified could involve further validation of those attributes, for example, by running multiple focus groups involving a broader range of participants with different health status, sociodemographic characteristics, and nationality. However, we reviewed a large amount of material to generate the list of attributes, and we therefore consider it unlikely that the result of such validation would differ much from the list proposed here. Our pilot study revealed differences between experts’ and patient group representatives’ views of the relative importance of some attributes. It would be necessary to repeat the study with larger samples of experts and patient group representatives to test the robustness of these differences. But the patient group representatives in our workshops represented a diverse range of rare diseases and all had experience at national and/or supranational levels of informing rare diseases policy. The clinical and health economics experts all had practical experience of informing payers and policymakers at national and/or supranational levels about rare disease value assessment. So, there is reason to believe that the different weights we found might persist even with the involvement of larger numbers of experts and patient group representatives.

Further research would be useful to determine the extent to which different groups’ ratings of a treatment against a particular benefit attribute were due to different understanding of the evidence about that attribute. Further workshops, or a DCE, could provide valuable insights by comparing the results from groups provided with different degrees of information and education about the rare diseases and OMPs to treat them.

To produce a single consensus set of weights within a jurisdiction would be a necessary step for implementation and hence deriving such a set of weights from a larger and fully representative sample of stakeholders is needed for each jurisdiction. We experienced great willingness by all participants within each of the workshops to listen to others’ views and arguments, to compromise, and to agree. We feel confident that adding a further stage to the process where, for example, the results from the two sets of participants were brought to a combined plenary meeting of all participants would be likely to yield a mutually acceptable set of weights.

It will also be important to elicit attributes’ weights from the general public (the ultimate funders of health care via taxation, social insurance, or private insurance) and alternative methodologies such as DCEs could be used for that purpose. There is a growing body of research using DCEs in this and other ways in health technology evaluations [21]. Estimating attributes’ weights across different countries will reveal how far preferences over treatment characteristics vary between health care payers and systems.

The aim of our study was to provide a framework to measure and value the benefits of OMPs. Decision-making processes involve a further step, which is beyond the scope of this short note, namely, the comparison of benefits with net costs to determine price, reimbursement status, and/or recommended use within a health care system.

Conclusions

Given the intrinsically complex nature of the rare diseases and OMPs environment, an MCDA approach for rare disease treatment value assessment has the merit of ensuring shared understanding of the elements of value as well as a clear articulation of trade-offs between those elements. We successfully piloted such an approach with patient group representatives and clinical and health economics experts who advise HTA bodies and payers. The MCDA approach offers a possible construct for more comprehensive guidance to HTA and P&R decision making.

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Supplemental Materials

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