New ISPOR Recommendations - Mapping Methods for Estimation of Health State Utility

This issue of Value in Health features an International Society for Pharmacoeconomics and Outcomes Research (ISPOR) Good Research Practices Task Force Report that makes recommendations for statistical methods for mapping (sometimes referred to as “cross-walking” or “transfer to utility” [1]) to estimate health utility (HU) from non-preference-based outcome measures. The report states the consensus position of the ISPOR Task Force on Good Practices for Outcomes Research–Use of Mapping to Estimate Utility Values From Non–Preference-Based Outcome Measures for Cost per QALY Economic Analysis.

This is the second task force report on HU estimation developed under the ISPOR Vision 2020 initiative, which identified “estimating health-state utilities for cost-effectiveness analysis” as one of the top two priorities for the development of good research practices guidance to address the lack of guidance in this area. The first such report was published in the September/October issue of Value in Health and made recommendations for estimating health-state utility for economic models in clinical studies [2].

HU data are estimates of the preference for a given state of health on a cardinal numeric scale, where a value of 1.0 represents full health, 0.0 represents dead, and negative values represent states worse than dead [3,4]. HU estimates are used in cost-utility analysis, a special type of cost-effectiveness analysis in which health benefits are usually measured in terms of quality-adjusted life-years (QALYs) [5], calculated by multiplying the number of years lived in each state of health by the HU estimate for each respective state [6].

Cost-utility analyses are increasingly used in many countries to establish whether the cost of a new intervention can be justified in terms of the health benefits that it offers. These decisions affect patient and physician access to treatments, product price, and, in turn, the return on investment in product development for manufacturers. HU estimates are typically among the most important and uncertain data inputs in cost-utility analyses, and poor quality health-state utility data result in greater uncertainty in decision making.

Recognizing that different HU estimates produced by different measurement methods and the need for consistency in decision making, many health technology assessment authorities have expressed a preference for a particular HU measure or measures. These are commonly generic, preference-based measures (PBMs; e.g., the EQ-5D) because they are applicable to a wide range of diseases, patients, and interventions. PBMs provide a means by which patients can record their own health state (e.g., by completing a questionnaire), and HU values that reflect preferences for health can be assigned to these health states (e.g., health states may be valued using time trade-off experiments in a sample of the general population). In cases in which clinical studies of the health technology of interest have not included a PBM, mapping is a means of estimating HU from data collected in the studies using a non-preference-based, patient-reported outcome measure (PROM).

Mapping requires a data set that includes patient responses to a PBM (e.g., the EQ-SD) and the PROM that was administered in the clinical study. This external data set is used to estimate a statistical relationship between the two outcome measures. This statistical relationship can then be used to estimate HU for the patients in the clinical study of the intervention, as it would have been measured using the PBM.

A large number of mapping studies have been reported, many of which have informed health technology assessments and decision making [7]. A variety of methods have been used, and alternative mapping methods have been reported to result in differing HU estimates and therefore differing cost-effectiveness estimates [8,9]. Particular care is needed in the selection or generation of an appropriate data set, in selection of the statistical model, and in assessment of model performance. In addition, careful reporting is important to allow for proper scrutiny of the methods and understanding of the strengths and limitations of the analysis and results.

The task force aimed to set out Good Research Practices for conducting mapping studies for use in cost-utility analyses. The recommendations encompass the selection of data sets for the mapping estimation; selection of the statistical model; and assessment of model performance, reporting standards, and use of results (including the appropriate reflection of variability and uncertainty).

These recommendations build on existing recommendations for mapping methodology [10–12] and address some gaps in these recommendations by taking an international perspective, covering additional aspects of mapping practice and reflecting contemporary state-of-the-art methods. As well as providing guidance for mapping to estimate HU for cost-utility analyses, the recommendations also have broader relevance for the estimation of preference-based outcomes as a function of other variables; for example, where HU is used as a measure of provider performance.

The task force suggests that, before mapping is undertaken, it is important to clarify the objectives of the analysis. Mapping is often performed to provide estimates for a specific cost-utility model. In this case, the objectives would be framed in terms of the HU estimates that will be needed for that model and developed by identifying the economic model health states, their definitions, and the requirements of the audience for the economic model (e.g., the decision-making body that will assess the results of the cost-utility analysis in which the mapping results are to be used). This information will help to inform the analyst’s choice of appropriate methods and data sets.

In the selection or generation of an appropriate data set, the task force cautions that mapping is unlikely to be successful if
there is little overlap between the concepts encompassed by the PROM and the PBM. A descriptive comparison of the content of the measures is recommended as a useful starting point. In terms of study design, observational studies may provide richer data for mapping studies than controlled clinical trials. Stringent inclusion and exclusion criteria applied in controlled trials often exclude patients who would be eligible for the treatment of interest in routine clinical practice (e.g., patients with comorbidities) and are therefore relevant for the economic analysis, and the period of follow-up may not capture more advanced disease states that are projected into the future in economic models (e.g., in progressive conditions). Data for these patients and health states may be more easily captured in an observational study. While statistical models may provide for extrapolation beyond the range of the available source data, the task force acknowledges that this is best avoided if possible.

The task force stresses the importance of understanding the statistical distribution of the data, careful model selection, and testing of model performance. No specific set of methods is advocated because the performance of different methods will vary according to the characteristics of the PBM, the disease and patient population in question, the nature of the explanatory clinical variables, and the form of intended use in the cost-utility analysis. The recommendations advocate use of a model type for which there is existing empirical evidence of good performance and which respects the key features of the PBM. The report concludes with recommendations on reporting of mapping studies and use of the results in cost-utility models.

The task force recommendations are expected to be relevant for analysts conducting mapping studies, peer-reviewers of these studies, researchers using the results of mapping studies in economic evaluations, and decision makers who act on the results. The guidance may be expected to make an important contribution to the field, advocating and informing the consistent application of appropriate analytical methods; appropriate use of the results in economic evaluations (including reflecting variability and uncertainty); informed scrutiny of methods; and an understanding of the strengths, limitations, and potential for bias in mapped HU estimates.

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