ISPOR Task Force Report: Estimating Health-State Utility for Economic Models in Clinical Studies

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The ISPOR Task Force Report "Estimating Health-State Utility for Economic Models in Clinical Studies: An ISPOR Good Research Practices Task Force Report," is one of two Task Force reports on health-utility 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. The second report considers mapping to estimate health-state utility from nonpreference based outcomes measures for cost per QALY economic analysis, and is expected to be published later in 2016/2017. The Task Force Report aims to provide helpful, practical advice for researchers planning the collection of health-utility data for economic modelling in clinical studies.

Cost-utility analyses, most often performed using economic models, are increasingly used in many countries to establish whether the cost of a new intervention can be justified in terms of health benefits. These decisions affect patients' and physicians' access to treatments, product price, and in turn, the return on investment in product development for manufacturers. Health-state utility (HSU) estimates are typically amongst the most important and uncertain data inputs in cost-utility models and poor quality HSU data result in greater uncertainty in decision making.

HSU 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 death [1,2]. HSU estimates are used in cost-utility analysis, a special case of cost-effectiveness analysis in which health benefits are usually measured in terms of quality-adjusted life years (QALYs) [3]. QALYs are calculated by multiplying the number of years lived in each state of health by the HSU estimate for each respective state [4]. For example, if an intervention confers 2 extra years of life at an HSU of 0.75, then the intervention would confer an additional 1.5 QALYs (2×0.75) to the patient.

The primary use of HSU estimates is for cost-utility analysis within health economic models. So, unlike other patient-reported outcomes (PROs), in most cases the aim is to generate utility estimates for economic model health states rather than to perform statistical comparisons between treatment arms. As HSU data may be collected via patient-completed forms, they are often considered as part of the PRO strategy, but their inclusion in a clinical trial or study requires special consideration to meet the needs of economic models.

The Task Force Report makes recommendations for the design of health-utility data collection in trials; the design of supplementary or alternative utility studies (including prospective and cross-sectional observational studies); and for statistical analyses and reporting. Selection of utility measures and optimisation of the timing of assessments to capture utility data for economic model health states and/or acute events (which have a short-term impact on quality of life) are discussed. Other issues considered include the mode of administration of the utility measure, special populations (e.g., patients who are unable to complete assessments), missing data, and generalisability of utility data collected in trials to economic model populations (i.e., patients expected to receive the intervention of interest in routine practice).

We recommend careful planning for HU estimation, starting early in the product development process, as there may be a need to conduct separate studies to supplement utility data collected in phase III trials (e.g., to provide HSU estimates for health states representing long-term disease progression which may not be captured within the follow-up period of Phase III trials). If there is uncertainty regarding appropriateness of utility measures in the condition of interest, qualitative and/or quantitative research may be needed to support instrument selection before the Phase III trial. In some cases, collection of data to allow development of a mapping algorithm may be needed. These studies take time, and Phase II trials may provide an opportunity to conduct some of this research, so some initial planning is recommended as early as Phase I (in parallel with Phase I studies) to make best use of opportunities across the trial program and to gather the information needed in time to design the HU data collection in Phase III trials. We hope that the guideline will help support health economics and outcomes researchers in designing health-utility data collection in clinical trials, optimizing methods to meet the needs of economic models, and planning any additional studies needed to supplement trial data.

ISPOR is offering a Short Course at the upcoming European meeting in Vienna (October 29, 2016) as an introduction and opportunity to discuss the guideline.

References

[1] Lenert L, Kaplan RM. Validity and interpretation of preference-based measures of health-related quality of life. Med Care 2000;38(Suppl. 9):II138-II150. [2] Feeny D. A utility approach to the assessment of health-related quality of life. Med Care 2000;38(Suppl. 9):II151-II154. [3] Robinson R. Cost-utility analysis. BMJ. 1993;307:859-862. [4] Kind P, Lafata JE, Matuszewski K, Raisch D. The use of QALYs in clinical and patient decision-making: Issues and prospects. Value Health 2009;12(Suppl. 1):S27-S30. ■

Additional information:
To view this Task Force report, go to: http://www.ispor.org/Estimating-Health-State-Utility-Economic-Models-Clinical-Studies-guidelines.aspe