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EDITORIAL

Decision Modeling for Cost-Utility Analysis

Cost-effectiveness analysis (CEA) provides estimates of health outcomes and costs of competing alternatives and is widely accepted as a useful tool for resource allocation. Health outcomes are commonly summarized as quality-adjusted life-years, which are a combination of quantity and quality of life. The rapid increase in CEAs expressed as cost per quality-adjusted life-year gained (known as cost-utility analyses)—and the increase in their use for health technology assessments (HTAs) and coverage decisions—underscores the need for sound recommendations for estimating health utilities to inform these analyses.

The International Society for Pharmacoeconomics and Outcomes Research Task Force on Good Research Practices for Outcomes Research—Collecting Health-State Utility Estimates for Economic Models in Clinical Studies was established to provide recommendations specifically for estimating health utilities when conducting cost-utility analyses using decision-analytic models. The task force report provides detailed guidance for estimating health utility values that are unbiased, relevant to the population being studied, and consistent with the preferences of the decision makers (e.g., HTA authorities) [1]. Of note for this editorial are 1) the recommended health-state utility measures, 2) the planning for and collection of health utility data, and 3) the role of models in CEAs in general and specifically when collecting health utility data from a clinical trial.

The recommended measure for estimating health-state utilities is to use a generic preference-based indirect assessment, such as the EuroQol five-dimensional questionnaire (EQ-5D), which is the preferred measure of many HTA authorities. Conducting a direct utility assessment with a standard gamble or time trade-off is essentially reserved as a last-resort option by the task force, despite the large body of literature that reports health utilities based on direct assessment (both for hypothetical health states and for a person's own health). When a standard gamble (or time trade-off) is used to directly assess hypothetical health states (using vignettes), one argument is that they do not capture the full distribution of outcomes experienced by patients if the vignettes are not based on a validated patient-reported health-related quality-of-life (HRQOL) measure (i.e., HRQOL measures that demonstrate content validity, construct validity, responsiveness, and reliability) [2]. In fact, vignettes that are not based on validated HRQOL measures do not meet the National Institute of Health and Care Excellence Methods Guidance for alternatives to the EQ-5D [2]. Although the task force report does not overtly place as high a bar on vignette descriptions, it seems to foreshadow a movement toward more rigorous requirements for vignette development than the traditional practice of developing vignettes on the basis of expert opinion. This would add substantially to the already onerous task of direct utility assessment, and the trade-offs between effort and return should be acknowledged and justified with appropriate evidence.

The task force report recommends early research activities that run parallel with the product development phase, such as conducting literature reviews and patient interviews at the time that phase 1 trials are under way. These early activities would no doubt be informative for developing economic models for those products entering phase 3 trials and beyond. There are, however, two aspects to this recommendation that deserve comment. First, it should be recognized that research conducted early in the product development phase may not end up being useful if the product is not approved. According to the US Food and Drug Administration, less than 25% of drugs that enter a phase 1 trial eventually end up in a phase 3 trial [3]. Thus, there should be thoughtful deliberation about the types of data collected in terms of the likeliness of it being useful (for a product under development or other approved or planned products). Second, the early research steps should be more explicit about what is entailed for the model conceptualization process, referring the reader to the ISPOR-SMDM Modeling Good Research Practices Task Force Report on conceptualizing a decision-analytic model [4]. It is common for the model conceptualization phase to come before, or at least be independent of, the data identification/collection phase.

The task force report distinguishes between health utility data collected in clinical studies and health-state utilities, the latter being what would be required for a decision-analytic model. If the health utility assessment tool is administered to all study participants, then that leaves the task of appropriately assigning study subjects to health states so that an average utility can be applied. Determining the health-state descriptions within a decision-analytic model is not trivial and deserves further discussion. The term "health state" is used both for one of the states defined by a health status classification system (HRQOL measure) of a generic preference-based model and for the disease- and intervention-specific health states of a decision-analytic model. Certainly, the health states of a decision-analytic model should not be based on the generic HRQOL system of, say, the EQ-5D, but should reflect that nature of the disease over time as well as the positive and negative impacts of the intervention. The task force report does correctly point out that "it is more appropriate that the health-utility data in a clinical trial be analyzed to inform [the] model, rather than be analyzed by treatment arm" [1, Section 6.1]. Furthering the discussion about how best to ensure that the health utility data collected in a clinical trial are most useful for informing health-state utilities in a model would be of value.

From a broader perspective, the decision to conduct a model-based versus a trial-based CEA warrants discussion. The task force report applies only to the collection of health-state utilities for economic models in clinical studies and is not focused on CEAs alongside clinical trials using statistical comparisons. This topic has been covered by another good practices task force [5]. What is missing is a broader discussion of factors that should be considered when deciding whether a model should be used in the

first place. I echo the sentiments voiced in one of the editorials accompanying the trial-based CEA task force report on the limitations of these studies [6]. In his editorial, Sculpher asserts that trial-based CEAs are often inconsistent with four principles of CEA, which, in turn, are principles that point to the need for decision-analytic modeling. Specifically, decision-analytic models allow the analyst to 1) extrapolate to population subgroups not observed in the trial, 2) extrapolate beyond the time horizon of the trial, 3) consider strategies that are relevant for the population but not evaluated in the clinical trial, and 4) synthesize data from disparate sources. The structure of a decision-analytic model provides the means with which empirical observations from different data sources and different study populations are used to inform the relevant measures for CEA.

Overall, the task force report covered a wide range of topics, from study design to analysis. The report focused on estimating health utilities specifically for economic models. Nevertheless, the focus on the development of a decision-analytic model, and its influence on study design and analysis, was limited.

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