



Risk in Perspective

The Importance of Modeling in the Economic Evaluation of Medical Technologies

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If there were unlimited resources to spend on health, then a reasonable approach for a society would be to implement any medical intervention that has an overall health benefit. Given that resources are limited, it is useful to know how various medical interventions compare with one another in terms of the amount of health benefit provided for each additional dollar spent.

Cost-effectiveness analysis (CEA) is an analytical tool that provides the means for a reasoned approach to the allocation of health-care dollars in light of constrained resources. CEA determines the optimal use of available medical technologies, where "optimal" means maximizing the health of a population for a given budget. Because there exist political, ethical, and legal issues that are relevant to the allocation of health care resources, CEA is offered only as an aid to policy makers and does not "make the decision."

Among the pioneers and practitioners of CEA, it is customary to use analytical structures—or mathematical models—to synthesize data on the costs and benefits of alternative clinical strategies. More recently, economic analyses have become integrated into the framework of clinical trials which previously focused on clinical outcomes alone. One of the controversies surrounding the application of CEA in this context involves the appropriate use of modeling techniques as a supplement to the primary data collection and analysis. Specifically, mathematical models are used to link data from multiple sources, extrapolate costs and health effects beyond the time horizon of a trial; and investigate how cost-effectiveness ratios might change if the values of key parameters in a model are changed.

This issue of **RISK IN PERSPECTIVE** discusses the use of CEA for the evaluation of medical technologies, and highlights the importance of modeling in this context.

The Use of Models in Cost-Effectiveness Analysis

In CEA, mathematical representations are often used to simulate the prognosis of a hypothetical cohort of patients under various treatment scenarios. The utility and properties of these

models have previously been demonstrated by decision analysts, systems analysts, and operations researchers. Of particular value to clinicians is the ability of a model to simulate a patient's life experience based on data from multiple sources including clinical trials, meta-analyses, observational databases, and expert opinion. Once the structure of a model is established, data on short- and long-term events (e.g., heart attack, cancer recurrence, or death), health-related quality of life, and costs are used to operationalize the structure. Typical outputs from a model are life expectancy, quality-adjusted life expectancy, and lifetime costs.

Recently, the Food and Drug Administration (FDA) issued draft guidelines on the regulation of commercial claims made about the cost-effectiveness of pharmaceuticals. While the guidelines recognize the value of CEA and embrace the use of randomized controlled trials to assess the cost-effectiveness of a drug, they seem to adopt an unfavorable view of the use of modeling. This stance toward modeling is of concern to practitioners of CEA. Clinical trials are designed to answer a specific clinical question and not to assess cost-effectiveness, and thus the use of models is often necessary to fill in the gaps when completing a CEA.

Importance of Models

Economic analyses are increasingly becoming standard adjuncts to clinical trials. Economic outcomes and health-related, quality-of-life measures are collected during the trial on all or a subset of the trial participants. Although this new source of data is useful in CEA, there are a number of situations where a modeling effort is warranted in the context of a clinical trial.

Clinical trials are often not designed to evaluate the long-term differences between two study groups of patients. For example, trials designed to evaluate therapies for patients who are positive for HIV often use a biological endpoint to measure effectiveness rather than disease per se. An example of a surrogate marker is the CD4 count in a patient's blood rather than mortality. It is widely accepted that a patient's CD4 count is a valid predictor of mortality and thus it is not always necessary to study patients all the way until death. In this

