

"Economic Evaluation in Clinical Trials, 2nd Edition"

Henry A. Glick and Jalpa A. Doshi, Seema S. Sonnad, and Daniel Polsky, 2015, 252 pages, Oxford University Press, \$59.95

Review by Norman M. Goldfarb

"Economic Evaluation in Clinical Trials, 2nd Edition" is a sophisticated handbook for building economic assessment into clinical research. A solid cost-benefit analysis is essential to justify the high price (and development cost) of many medicines and medical devices.

This book has been selected for
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Essential reading for clinical research professionals

The following excerpt explains three ways of obtaining QALY (Quality of Life Years) measures from study participants:

Direct elicitation from participants

A second common approach for assessing QALYs in trials is to directly elicit preferences from study participants. The three most common methods for doing so are the standard gamble, the time trade-off, and the rating scale. Rasanen et al. have reported that, among studies that have directly elicited preferences from patients as part of an economic evaluation, the standard gamble has been used 21% of the time, the time trade-off has been used 42% of the time, and the rating scale 37%. These numbers vary slightly from those previously reported by Morimoto and Fukui.

Standard gamble

A standard gamble asks participants to trade off a certain, intermediate outcome for a gamble for a better and worse outcome. For example, the participant might be asked to choose between living with current health for 10 years versus a $p/1-p$ gamble of living with full function for 10 years or dying immediately. Standard gambles satisfy the axioms of expected utility theory as proposed by von Neumann and Morgenstern. This theory states that, when making decisions based on maximization of expected utility, the measures of outcome should reflect preference measured with risk. As Drummond et al. have noted, in addition to being risk based, standard gambles — unlike rating scales — also require that participants choose between health outcomes.

While there are a number of methods available for framing a standard gamble, the most common one used for direct elicitation of preference for current health poses what is referred to as a probability-equivalent standard gamble. One approach for presenting a probability-equivalent gamble is to describe a level of health for a specific length of survival, for example, current health for 10 years or for the number of years that represent the participant's life expectancy versus a gamble for best and worst imaginable health for the same number of years. Alternately, some authors omit the description of the length of survival and offer current health with length of survival unspecified versus a gamble for best and worst imaginable health. In either case, the participant is asked to identify p such that she is indifferent between the certain outcome and the gamble.

We interpret the probability that makes the respondent indifferent between the two choices as the preference or utility score. We do so because, by indicating indifference, the respondent states that the utility of the certain outcome is identical to the utility of the gamble. Under expected utility theory, the utility of the gamble is made up of p times the utility of the best outcome, for example, full functioning, which we assume has a utility of 1.0, plus $1-p$ times the utility of the worst outcome, which drops out of the equation because we assume its utility equals 0.

Time trade-off

A time trade off asks participants to trade off morbid years for healthy years. A participant is asked to choose between living some length of time — for example, 10 years or the number of years that represent the participant's life expectancy — with her current health versus living a shorter period of time with fully functional health. The participant is asked to identify the number of years with fully functional health that makes her indifferent between the longer morbid life expectancy and the shorter fully functional life expectancy.

Unlike standard gambles, time trade-offs do not satisfy the axioms of expected utility theory because they are not measured with risk. Like standard gambles, they do require that participants choose between health outcomes.

To obtain a preference score, we divide the number of healthy years identified by the participant by the number of years with current health. For example, if the participant reported that she was indifferent between 7 healthy years and 10 years with current health, the resulting preference score would be 0.7 (7/10).

Rating scale

A rating scale — also referred to as visual analog scale or feeling thermometer — asks participants to rate how good or bad their current health is on a 0-1 or 0-100 scale. Zero often represents worst imaginable health or death; 1 often represents “best imaginable health” or “full health.” The EuroQol 100-point visual analog scale is a commonly used visual analog scale. It is drawn as a 20-cm vertical line, with its lower end representing the worst imaginable health state (0) and its upper end representing the best imaginable health state (100). It is subdivided into 10-unit intervals with subinterval tick marks. More generally, as Green et al. have noted, rating scales can vary in presentation in terms of length of the line, whether they are drawn vertically or horizontally, and whether or not they have intervals marked out with numbers. Some have argued that having intervals marked out with numbers can induce memory effects and clustering.

Rating scales neither satisfy the axioms of expected utility theory, nor require that participants choose between health outcomes.

If a rating scale ranges between 0 and 1, the point on the line selected by the participant represents her preference score; if the scale ranges between 0 and 100, the point on the line divided by 100 represents this score.

The book consists of 11 chapters:

- Introduction to economic evaluations in clinical trials
- Designing economic evaluations in clinical trials
- Valuing medical service use
- Assessing quality-adjusted life years
- Analyzing cost

- Analyzing censored cost
- Comparing cost and effect: point estimates for cost-effectiveness ratios and net monetary benefit
- Understanding sampling uncertainty: the concepts
- Sampling uncertainty: calculation, sample size and power, and decision criteria
- Transferability of the results from trials
- Relevance of trial-based economic analyses

The book is available in bookstores.

Reviewer

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