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Economic studies part I: basics and terms

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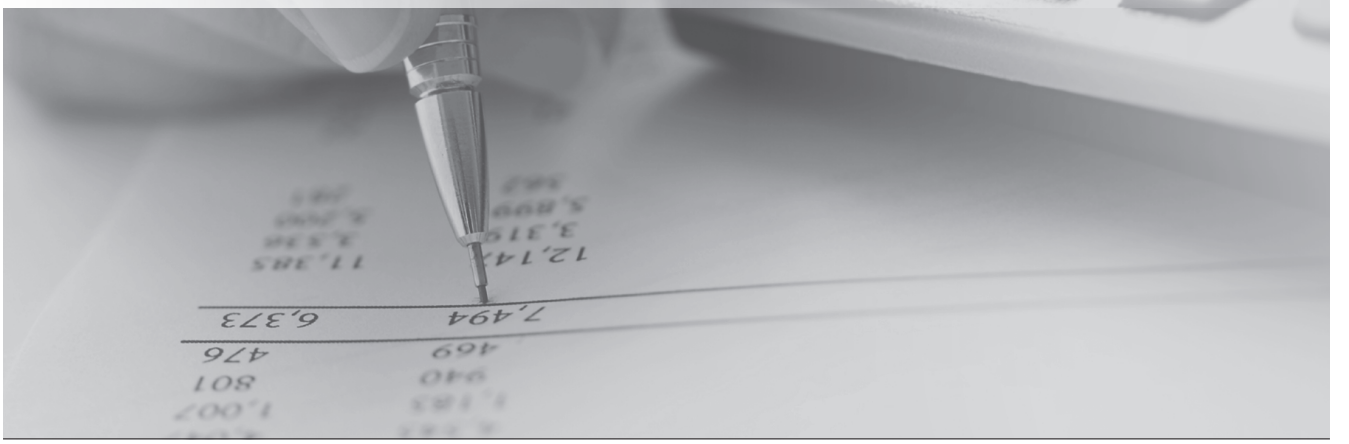


There is an increasing realization across the globe that the resources available in society for the provision of medical care are finite. As such, in addition to establishing the efficacy and effectiveness of spinal procedures, it is becoming increasingly important to also describe their economic impact. In general, the most compelling analyses are those which compare the costs and efficacy of two competing options for care in a formal, full economic analysis.

In this article we describe: (1) some terms you may encounter when reading economic studies, (2) the most often-used types of economic studies, and (3) some considerations for economic evaluations of spinal procedures.

Terminology

Before describing the types of economic analysis, it is important to have a sense of the terminology often used in these types of studies. An understanding of the terms and concepts below is crucial for assessing the usefulness and quality of economic evaluations.

Use of alternatives: Appropriately structured economic studies will clearly state the intervention under study and the intervention it is being compared to—the comparator.

Clinically appropriate intervention/comparator: Economic evaluations should state clearly the components of both intervention and comparator. Since cost-effectiveness ratios are derived using alternatives, it is crucial that both intervention and comparator be realistic, appropriate reflections of actual clinical use. For example, comparing procedure B conducted in people older than 70 years may or may not be a clinically appropriate comparison group for procedure A conducted in people younger than 30 years. Components should include the surgical procedure, any details of how it was conducted, characteristics of the patient population, and time of follow-up. For each procedure, outcomes related to efficacy or effectiveness as well as rates of complications, sequelae, and additional procedures that may occur along the clinical course also need to be articulated.

Perspective: The perspective of an economic evaluation has direct bearing on the types of costs and outcomes included in the evaluation. A patient perspective would consider only costs and outcomes relevant to patients such as out-of-pocket costs; a payer perspective would entail primarily assessing the charges relevant to health insurance payers, such as procedures, visits, and rehabilitation and hospital services. A provider perspective would highlight direct costs to provider groups, clinics, or hospitals such as equipment and facilities costs and staff time. A health system perspective would entail all costs and outcomes associated with providing care, regardless of where costs are incurred. A societal perspective is the most comprehensive and arguably most closely approximates the perspective of policymakers who would use the results of these studies. Such studies strive to include all outcomes and costs to health systems and patients, both long-term outcomes and adverse events, and both direct costs of intervention and costs to patients, including lost productive time, caregiver and travel time, and recovery or recurrence/relapse.

Time horizon: Economic evaluations often use modeling techniques to estimate the costs and outcomes of alternative interventions over time. Therefore, an appropriate time horizon is necessary, consistent with the perspective of the study and the natural history of the illness in question. Normally, a long-term time horizon of at least 10 years is preferable, one that can capture all relevant sequelae and relapse, reoperation, or recurrence; although in some cases a shorter time horizon can also provide valuable information. For longer time horizons, human tendencies to prefer immediate benefits over future ones should be described in the study assumptions and modeled appropriately, commonly using discounting of costs and benefits beyond 1 year of around 3–6% annually.

Data sources: Economic evaluations most often compile data on the effectiveness of the interventions, their costs, and the impact on patients (utility) from a variety of sources. High-quality economic evaluations use the best available data, usually patient level, clinical and cost data collected alongside a randomized controlled trial. If these data are not available or if randomized trial data is incomplete or includes only short-term outcomes, then data from high-quality observational studies (eg, methodologically rigorous prospective cohort studies that minimize bias) can provide useful information for modeling. Existing databases of reasonable completeness can provide cost estimates as well. Expert opinion—providers' own estimates of clinical or cost estimates—is often biased and therefore considered the least reliable source of data.

Utility: Health economists use utility to estimate the health impact of a condition before and after an intervention. It is expressed as a number between 0 and 1, where 1 indicates perfect health and 0 indicates death. In a hypothetical example of an intervention for back pain, the utility before intervention might be 0.5, and improve to 0.8 after intervention. Utilities are ideally calculated from surveys of people who have direct experience with the condition (patient reported), but are sometimes estimated by clinicians or extrapolated from other studies. Utilities are calculated ideally using various patient-reported outcomes, such as a Visual Analog Scale for pain or validated assessments of health-related quality of life. Conditions associated with pain are often considered to have low utility.

Sensitivity analysis: Since economic evaluations use diverse data sources and assumptions, authors should report sensitivity analyses to assess uncertainty around the results. One-way sensitivity analyses vary individual variables (such as reoperation rate, pain

reduction, or procedure cost) across a range to see how the end results change. Probabilistic sensitivity analyses use bootstrapping or other statistical techniques to arrive at a sense of which variables are driving the model results. This can be useful to decision-makers who need to understand the most important components of the cost and how they impact the cost-effectiveness.

Incremental cost-effectiveness ratio (ICER): An ICER describes the results of an economic evaluation. When the intervention of interest is more effective and more costly than the comparison intervention (**Fig 1**), the cost-effectiveness ratio provides an estimate of the cost per unit of improvement—for example, $80,000 per quality-adjusted life year.

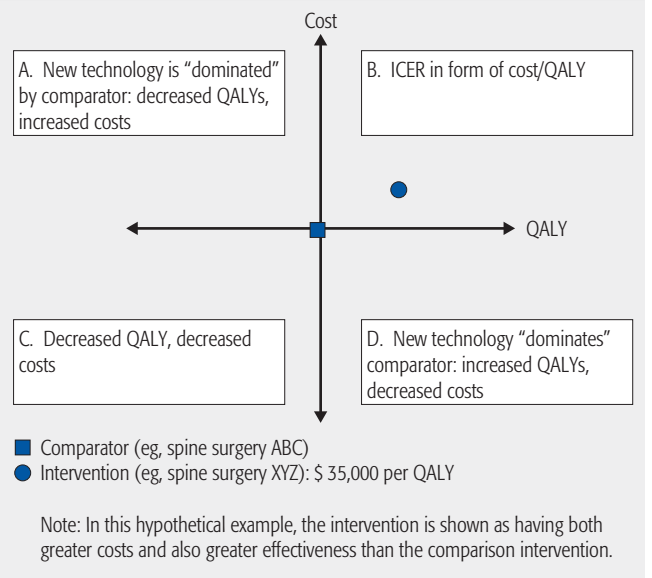
Types of economic studies

Studies that report only costs or cost of illness are considered "cost-only" studies. These studies can serve as important sources of data to inform economic evaluation but are not themselves considered economic evaluations.

Full economic evaluations identify and compare appropriate alternatives, their incremental impact on health outcomes, and their incremental costs. They compare two well-defined clinical alternatives in the form of an ICER, broadly defined as the cost per unit of clinical improvement—derived by dividing the differences in effectiveness by the difference in costs:

Difference in cost/difference in effectiveness

Fig 1 Cost-effectiveness plane.



There are several types of full economic evaluations (**Table 1**).

- Cost-minimization studies consider the cost differences between alternatives of assumed or established equal effectiveness.
- Cost-benefit studies consider both costs and benefits in monetary terms.
- Cost-effectiveness studies consider differences in costs and differences in effectiveness, but effectiveness is measured variably between studies (eg, can be survival or a condition-specific outcome, such as symptom-free days).
- Cost-utility studies consider differences in costs and outcomes for quality-adjusted survival, most often using the quality-adjusted life year (QALY). Cost-utility studies have the advantage of providing an ICER expressed as "cost per quality-adjusted life year" (cost per QALY) that eases comparison across multiple studies (**Table 1**). Cost-utility studies are usually considered the gold standard for economic evaluation but are not common in spine treatment literature overall.

Interpreting results of economic studies: When reading an economic evaluation, many people just want to know the bottom line: "Is the intervention cost-effective or not?" However, there is rarely a simple answer. Economic evaluations are designed to provide information on various interventions that should be interpreted by policymakers using the values and assumptions that reflect the constituents they represent. These assumptions include the perspective, time horizon, and patient centeredness. Decisions may also reflect a "threshold" at which an intervention is considered cost-effective. For example, a country or regional government may have a number—such as $50,000 per QALY—that falls below the perceived cost-effectiveness of an intervention. Or, decision-makers may assess cost-effectiveness considering the prevalence or severity of the condition in question.

The grid in **Fig 1** shows a visual way to interpret the results of an economic study that is often used in teaching health economics. Relative to the comparator intervention, a new intervention can either be more effective and less costly ("dominates" the comparator—quadrant A) or less effective and more costly (is "dominated"—quadrant D). If the intervention is less effective and less costly, policy discussions may ensue about resource allocation (quadrant C). If the intervention is more effective and more costly (quadrant B), an ICER is generated that can be compared with other interventions. Only interventions that are both more effective and more costly are represented with an ICER. In **Fig 1**,

Table 1 Summary of study types.

	Effectiveness measure	Outcome	Notes
Cost studies			
Cost	None	Cost of procedural, treatment, or other charges	Inform full economic evaluations
Cost of illness or economic burden	None	Includes indirect, patient, society costs	Inform economic evaluations
Full economic evaluations			
Cost minimization	Assumes equal effectiveness between alternatives	Difference in cost	Alternatives are rarely truly equal in effectiveness
Cost benefit	Costs of effects (benefits), costs of intervention	Net benefits; cost-benefit ratio; willingness to pay	Controversial to express benefits in monetary terms
Cost- effectiveness	Natural or condition-appropriate measure (eg, survival; pain reduction; time to recurrence)	Cost per improved outcome	Most useful for within-health state comparisons; less so for comparing studies of different health states
Cost utility	Quality-adjusted survival (QALY; DALY)	Cost per QALY (or cost per DALY)	Easiest to compare across studies; reflects more assumptions about utility

a hypothetical example of a new surgical procedure is plotted in the upper-right quadrant with a cost-effectiveness ratio. Some single-payer systems have an implied "threshold" of cost-effectiveness under which interventions are considered "cost effective" (eg, $50,000 per QALY), but this is controversial. The emphasis that policymakers may place on cost-effectiveness and thresholds of this type may vary across policy-making bodies and countries, and include consideration of competing priorities for resources and the quality of the economic study. Ideally, full economic analyses are only one data component of the policy decision-making process.

Economic evaluations in spine surgery

Economic evaluations of interventions that involve spine surgery face unique challenges. The goal of surgery is often pain reduction or improved function rather than survival, so measures of quality-adjusted survival may be less sensitive in this setting. Similarly, early pain reduction relative to a comparison intervention (eg, pain relief after 2 weeks with procedure A compared with 6 weeks with procedure B) may be extremely relevant to an individual patient but over a long-term time horizon may no longer be detectable as a benefit. Therefore, patient perspectives on the benefits of intervention (patient-centeredness) are crucial.

Summary

Economic analyses have become an increasingly important consideration in formulating health policy in most countries around the world. Thus, it has become increasingly important for clinicians to enhance their understanding of such analyses. In general, cost studies provide limited information for policy development, and full economic evaluations should be presented. When well done, economic analyses provide insight into how scarce health-care resources might be best used in a specific setting but should not be used in isolation. As with any study type, they have their limitations and it is important to be able to put the results within the context of study quality. Many concepts described in this article (eg, use of appropriate comparator, performing sensitivity analysis) are an important part of the critical appraisal of such studies. The next part of this series deals with the assessment of the quality of economic analyses.

Recommended readings about economic evaluation:

Drummond MF, Sculpher MJ, Torrance GW, et al (2005) *Methods for the Economic Evaluation of Health Care Programmes.* New York: Oxford University Press.

Centers for Disease Control and Prevention: What is an economic evaluation? Introduction to economic evaluation. Available at: www.cdc.gov/owcd/EET/SeriesIntroduction/1.html

Health Economics Information Resources: A self-study course. National Information Center on Health Services Research and Health Care Technology (NICHSR). Available at: www.nlm.nih.gov/nichsr/edu/healthecon/beginningend.pdf

Boos N (2009) The impact of economic evaluation on quality management in spine surgery. *Eur Spine J;* 18(3 Suppl):338–347.

Holtz A, Nelson HD, Reid E (2012) Our questions, our decisions: standards for patient-centered outcomes research. *Preliminary Draft Methodology Report*, PCORI Methodology Committee; Patient-Centered Outcomes Research Institute. Available at: *www.pcori.org/assets/Preliminary-Draft-Methodology-Report.pdf*