

Assessing Research Protocols: Economic Evaluations

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Definition of economic evaluation:

The systematic and explicit comparative analysis of alternative courses of action in terms of both their costs and consequences.

Basic task is to identify, measure, value, and compare the costs and consequences of the alternatives being considered. Provide information to help answer questions of efficiency.

What they are:

1. Deal with both the inputs and outputs (costs and consequences) of activities. The linkage of costs and consequences allow us to reach our decision.
2. Are concerned with choices. Resources are scarce so choices must be made, sometimes based on many criteria. Economic analysis strives to identify and make explicit one set of criteria that may be useful in deciding among different uses for scarce resources.

Is it truly an economic evaluation?

Table 1: Distinguishing characteristics of health care evaluation (from Drummond et al. (2005))

| | | <i>Are both costs (inputs) and consequences (outputs) of the alternatives examined?</i> | | |
|--------------------------------------------------|----------------------------|-----------------------------------------------------------------------------------------|----------------------------------------------------------------------|-----------------------------------|
| | | No | | Yes |
| <i>Are two or more alternatives compared?</i> | No | Examines only consequences | Examines only costs | |
| | | 1A Partial evaluation 1B | | 2 Partial evaluation |
| | <u>Outcome description</u> | <u>Cost description</u> (e.g., cost of illness, burden of illness) | <u>Cost-outcome description</u> | |
| | Yes | 3A Partial evaluation 3B | | 4 Full economic evaluation |
| Efficacy or effectiveness evaluation (e.g., RCT) | | Cost analysis | Cost-effectiveness analysis Cost-utility analysis Cost-benefit | |

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|--|--|--|----------|
| | | | analysis |
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Is the correct type of economic evaluation being used given the question to be answered?

The type(s) of evaluation used for the economic evaluation should be clear with a reasonable justification of how the chosen type helps to answer the research questions posed.

Table 2: Measurement of costs and consequences in economic evaluation (adapted from Drummond et al. (2005))

| Type of study | Measurement of costs | Identification of consequences | Measurement/valuation of consequences | Questions that can be answered |
|--------------------------------------------------------------------------|----------------------|---------------------------------------------------------------------------------------------------------------------------|-------------------------------------------------------------------------------------------------------------|--------------------------------------------------------------------------------------------------------------------------|
| Cost-effectiveness analysis (CEA) | Monetary units | Single effect of interest, common to both alternatives, but achieved to different degrees. One dimensional effectiveness. | Natural units (e.g., life-years gained, disability-days saved, points of blood pressure reduction, etc.) | What is the cost per natural unit (e.g., strokes avoided)? |
| Cost-utility analysis (CUA) | Monetary units | Single or multiple effects, not necessarily common to both alternatives. Multidimensional effectiveness. | Healthy years or health state preference values (typically measured as quality-adjusted life-years (QALYs)) | What is the cost of gaining the improvement in health state? |
| Cost-benefit analysis (CBA) | Monetary units | Single or multiple effects, not necessarily common to both alternatives. Multidimensional effectiveness. | Monetary units | Is it worth achieving this health care goal? What level of resources should we allocate to this health care goal? |
| Cost-minimization analysis (CMA) <i>Only a PARTIAL evaluation</i> | Monetary units | Previous research has shown equal effectiveness or two therapies use near-identical technology | None | Given that we want to achieve this health care goal, which options would cost the least? |

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| | | | | |
|---------------------------------------------------|----------------|------|------|---------------------------------------------------------------------|
| | | | | What is the most efficient way to spend a given health care budget? |
| Cost analysis <i>Only a PARTIAL evaluation</i> | Monetary units | None | None | What is the cost of delivering a health care intervention? |

CHEERS statement (checklist) and Drummond et al. (2005):

<http://www.ispor.org/workpaper/CHEERS/revised-CHEERS-Checklist-Oct13.pdf>

1. **TITLE:** Title identifies the study as an economic evaluation or uses more specific terms such as “cost-effectiveness analysis” and describes the interventions being compared.

Who needs this study and why?

2. **BACKGROUND & OBJECTIVES:** Background provides an explicit statement of the broader context for the study and its relevance for health policy or practice decisions.
3. **TARGET POPULATION:** The base population and subgroups to be analyzed are described, including a rationale for why they were chosen.

In many cases, the results of economic evaluations will vary by population characteristics

4. **SETTING & LOCATION:** Relevant aspects of the system(s) in which the decision(s) need(s) to be made are provided.

An economic evaluation addresses a question relevant to the place and setting in which the resource allocation decision is being contemplated. A clear description of the location, setting, or other relevant aspects of the system in which the intervention is provided is needed so that readers can assess external validity, generalizability, and transferability of study results to their particular setting.

5. **STUDY PERSPECTIVE:** The perspective or viewpoint of the study is provided and related to the costs being evaluated.

Important to consider because costs and benefits may accrue beyond the patient, or shift to different areas with a new program/intervention. May want to explicitly consider how costs shift as a result of a particular health care option.

Because perspectives lack standard definitions, authors should describe the perspective (e.g., health care system, societal) in terms of costs included and their associated components (e.g., direct medical costs, direct nonmedical costs, and indirect/productivity costs), and how this fits the needs of the target audience(s) and decision problem.

Some examples are:

- **Health System/Payer** – direct medical care costs, including the cost of the intervention itself and follow-up treatment costs

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- **Health care sector** – all costs that fall to the health sector, including, but not limited to, hospitals, specialists, primary care physicians, and community services. Does not include informal carer costs, patient transport costs or time off work.
 - **Government** – all costs and consequences to the government
 - **Third party payer** – all costs and consequences to a third party payer
- **Societal** – Includes health system/payer and also estimates broader costs to society (e.g., productivity losses resulting from poor health or premature death, family costs, or costs to other sectors such as the criminal justice system). Considers all relevant costs and consequences no matter who pays or receives benefits from them.
 - **Health care unit/organization** – e.g., a hospital. Would include all medical and non-medical costs, including supplies, clinician time, and relevant overhead costs.
 - **Patient** – all costs and consequences to a patient, including the opportunity cost of patient's time.
 - **Family** – all costs and consequences to family (or informal care givers)
6. **STUDY QUESTION:** Identifies the type of economic evaluation, examines both costs and consequences, and requires details of the study (patient) population, the intervention of interest, the relevant comparator(s), and the health care setting.

How were the alternatives for consideration chosen?

7. **COMPARATORS:** The interventions or strategies being compared are described and the rationale for their choice is provided.

Interventions and delivery of technologies may differ among countries or settings, making it important to describe the relevant characteristics of studied interventions. The clinician on the team is important for this step, as is the existing literature.

Interventions considered should include all relevant ones, including “do nothing,” “current practice,” or “the most cost-effective alternative.” The relevant interventions can differ depending on whether the treatment/service is delivered in an urban vs rural setting.

There should be a clear description of each intervention/strategy. This includes intensity or frequency of treatment (for behavioral or nondrug interventions), drug dosage schedule, route, and duration of administration.

- What is done (screening program, treatment, etc.)
- To whom (patient population)
- How (process / resources to treat, screen, etc.)

8. **OUTCOMES/CONSEQUENCES:** The outcomes chosen as measures of benefit are described and their relevance for the analysis being performed is provided.

The findings of an economic evaluation may be sensitive to the choice of outcome, the reason for choosing one measure of outcome over another should be provided.

Health consequences

<https://www.nhmrc.gov.au/files/nhmrc/publications/attachments/cp73.pdf>:

- Patient-relevant: Outcomes that matter to the patient and their carers. They need to be outcomes that patients can experience and that they care about (e.g., quality of life, return to normal function)
- Clinical: Outcomes defined on the basis of the disease being studied (e.g., survival in cancer)
- Surrogate: A laboratory measure or physical sign used as a substitute for a clinically meaningful endpoint that measures directly how a patient feels, functions or survives.

Preferences: In the cast of a *cost-utility analysis*, the population from which preference valuations will be obtained should be described in terms of size and demographic characteristics. For example, a representative sample of the general population, patients, providers, and/or expert opinion. This population may differ from the study population for the economic evaluation.

Methods used to measure utilities:

1. Direct (e.g., Time trade-off, Standard Gamble, Visual Analogue Scale)
2. Indirect (e.g., EQ-5D (Euroqol), Health Utilities Index (HUI), Quality of Well-Being (QWB) scale, Short Form health survey (SF-36))

What is known about the effectiveness of the alternatives chosen?

9. **MEASUREMENT OF EFFECTIVENESS:** The source(s) of clinical effectiveness data is (are) provided and methods of identifying and synthesizing relevant sources of data is provided.

If the economic evaluation is based on a single experimental or nonexperimental study with patient-level data, the design features of that source study or reference should be provided. If the economic evaluation is a synthesis-based economic evaluation, it will require adequate information about the systematic review or meta-analysis, or a reference to a report.

What is known about the likely costs and funding implications of the proposed alternatives?

10. **COSTS & RESOURCES:** The approach to estimate resource use for the interventions being compared or health stated used in modelling is described, including

adjustments made to approximate opportunity costs. The currency and if necessary, the methods to convert costs into a common currency are provided.

Costing involves: 1) estimation of the resource quantities in natural units and 2) the application of prices (unit costs) to each resource item. The various costs used and the sources for the estimation of resource quantities and prices (unit costs) should be outlined.

Flow-on effects: Including side-effects. These are resources used (or saved) associated with the health care options, irrespective of who pays for them. Flow-on effects are the predictable occurrences that happen as a result of the health care option.

Is differential timing considered in the proposed study for costs and consequences?

11. TIME HORIZON: Time horizon(s) for costs and consequences is (are) provided and their appropriateness justified. It can be short (e.g., antibiotics for a throat infection) or lifetime (e.g., preventative interventions or a chronic disease treatment).

This is the length of time over which costs and consequences are being evaluated. It reflects the long-term consequences of a decision and is typically longer than the length of follow-up in clinical trials. Time frame for cost and benefits must be the same.

If the time horizon is shortened for practical reasons this decision should be justified (an estimation of any possible bias should be given). Even so, it should be long enough to capture all relevant costs and consequences of the health care option (especially when patient-level outcomes are considered).

12. DISCOUNT RATE: The chosen discount rate is provided and explained why it is appropriate.

Reporting discount rates is important because the findings of an economic evaluation may be sensitive to the chosen discount rate. Discount rates are required when costs or consequences of an intervention are not realized for several years. The chosen rate can be chosen based on local economic evaluation guidelines (refer to CADTH website: http://cadth.ca/media/pdf/186_EconomicGuidelines_e.pdf. In Canada the standard rate is 5% with sensitivity analyses using 0% and 3%).

Discount rates are often not used in economic evaluations with time horizons less than a year. Even so, the discount rate of 0% should be reported for clarity.

Does the proposal identify other methodological considerations?

13. **DECISION ANALYTIC MODEL:** If a decision analysis is carried out the specific type of model used is identified (e.g., decision tree or markov model) and any assumptions that need to be made.

The proposal should explain why it is appropriate to use the particular model in the study. This explanation might refer to the similarity of the model structure to models used in previous economic evaluations. For more information on good practices in modeling research please refer to the ISPOR website:

<http://www.ispor.org/taskforces/GRPModelingTf.asp>

14. **SENSITIVITY ANALYSIS:** The proposal indicates that a sensitivity analysis will be carried out on variables with uncertainty and for any assumptions.

Discount rates, unit cost vectors, and study perspective are areas where sensitivity analysis can be done to capture uncertainty around these parameters.

Definitions:

Cost-shifting: Occurs when a cost borne by one section of society in the health care of a medical condition is placed upon another. For example, early discharge after operation can shift costs from the hospital to the patient, patient's family and community medical services. This is important to recognize when carrying out an economic evaluation to ensure efficient decisions on the provision of care.

Cost-effectiveness analysis (CEA): A type of economic evaluation that compares options that have a common health outcome. The output is generally displayed as cost per unit of effect. Useful when making decisions between limited options within a given field and within a given budget. Aids decisions between disease-specific interventions/treatments.

Cost-utility analysis (CUA): This type of economic evaluation utilizes a form of utility measure (e.g., QALY) to value the outcomes of a program or intervention in order to aid decisions based on an existing budget.

Cost-benefit analysis (CBA): Measures costs and benefits (value) in monetary amounts. This allows comparisons to be made between programs both in and out of the health sector, aids decisions that involve expanding the current budget to accommodate a new program, and is potentially the broadest form of economic evaluation.

Discount rate: A rate used to convert the value of future costs and benefits (or consequences) into their present values. Discount rates are usually between 3% and 5%.

Incremental cost-effectiveness: The difference between the cost-effectiveness of two programs when one moves from one program to the other. The incremental cost-effectiveness ratio (ICER) is the difference in an outcome measure divided by the difference in price and examines extra consequences and costs one program imposes over the other.

Margin: The extra or incremental costs or consequences of each option when compared to the other.

Marginal benefit: The extra benefit received for the consumption of one more unit of a health care program or treatment.

Marginal cost: The extra cost of producing one more unit of the health care program or intervention.

Opportunity cost: The opportunity cost is what must be given up in order to obtain something (i.e., the value of time or any other input in its highest value use).

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Opportunity cost is the benefits forgone because the next best use was not selected (Gold et al 1996). Opportunity cost captures the notion of scarcity.

QALY: Quality-adjusted life-year. A generic (or common) outcome measure that takes into account both quality of life and length of life (mortality).

Sensitivity analysis: Tests whether variations in the assumptions made affect the conclusion of an economic evaluation. It is carried out by varying the items about which there is uncertainty over a specified range.

Utility: A technical term used by economists to denote satisfaction or well-being. It is generally used to show the preferences individuals or society may have for any particular set of outcomes or health state.

What they are not:

From: <http://www.cihr-irsc.gc.ca/e/45336.html#a1.4>

Evaluation: (Scriven, 1991) – the process of determining the merit, worth, or value of something, or the product of that process (p.139).

Evaluation research: purpose is to create new knowledge. (what can be learned about...? Are the theorized benefits being realized?)

Developmental evaluation: purpose is to help support the design and development of a program or organization. This form of evaluation is particularly helpful in rapidly evolving situations. (questions of “what is the best way to...”)

Formative evaluation: purpose is to refine or improve a program. Conducted in the development or implementation stages of an existing initiative. (questions of “improvement” or how should something be implemented)

Summative evaluation: purpose is to make a judgment about the value or worth of a program or activity at the end of the program activities, usually focuses on outcomes. This type of evaluation is conducted primarily for reporting or decision-making purposes. (questions of “should” a program be adopted, is it better, should it be expanded and funded?)

Program evaluation: Undertaken to inform program management decisions.

Performance measurement: is a planning and managerial tool. Focuses on results, most often measured by a limited set of quantitative indicators. Pre-post measurement designs.

References:

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<http://www.ispor.org/taskforces/GRPModelingTf.asp>

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<http://www.ispor.org/ValueInHealth/ShowValueInHealth.aspx?issue=3D35FDBC-D569-431D-8C27-378B8F99EC67> . Checklist available from:
<http://www.ispor.org/workpaper/CHEERS/revised-CHEERS-Checklist-Oct13.pdf>

Other Resources:

ISPOR – Assessing the Evidence (for Health Care Decision Makers)
Public online/interactive questionnaires to evaluate modeling, observational, and network and health care decision making aimed at improving patient outcomes.
<https://www.healthstudyassessment.org/Introduction>