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A guide to health economic evaluations

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Abstract

Economic evaluations are important in healthcare because they help to inform decisions on allocating resources within cash-limited systems. Such evaluations are increasingly carried out in appraisals of new treatments, such as those by the National Institute for Health and Clinical Excellence (NICE). Healthcare professionals and others need a working knowledge of health economic evaluations to assess or reach decisions informed by results from such calculations. Here we discuss different types of health economic evaluations and how the calculations from these analyses are used in decision-making.

Key terms and concepts

- Efficacy and effectiveness

The terms ‘efficacy’ and ‘effectiveness’ are often used interchangeably when referring to the extent to which an intervention offers benefit. In reality, however, there are significant differences between the two. Efficacy is a measure of the benefit (if any) of an intervention when it is applied under idealized, tightly controlled situations such as in randomised controlled trials.1,3 Demonstration of benefit in such trials is evidence that the intervention has the potential to work, at least when used under the conditions specified in the research. By contrast, effectiveness is used to describe the benefit achieved (if any) when the intervention is used in everyday practice (i.e. typically in a more representative population than in randomised controlled trials). The efficacy seen in randomised controlled trials is often greater than the effectiveness seen in routine practice. Potential explanations for this include the fact that trials may exclude patients with other diseases or conditions that might interfere with the intervention studied, or because the study inclusion criteria lead to recruitment of those likely to adhere to the trial's protocol. Also, the clinicians involved in the trials are selected on the basis of interest and expertise, which may not be replicated in routine practice. In addition, the trial setting itself may provide a more supportive environment for patients than is typically found in clinical practice.

- Comparators and uncertainties

In a health economic evaluation, the appropriate comparator is a therapy or care package (preferably the optimal or gold standard therapy) that is most likely to be displaced by adoption of the new treatment.2 However, comparators chosen for use in clinical trials may not be those used in everyday practice nor the optimal or gold standard intervention; also, what is considered to be the gold standard intervention for use as a comparator may change over time. As a result of such factors, the most appropriate data for conducting a health economic analysis for a particular setting may be unavailable. Therefore, such evaluations sometimes rely on the extrapolation of results from trials in other settings.2 This inevitably introduces uncertainties, since the evaluation will have to make assumptions about how the available data relate to the setting of interest. One way to help understand the impact of such assumptions is to test what effect changes in the assumptions have on the calculated results (a sensitivity analysis).

- Quality-adjusted life-year

Whether and by how much intervention extends life is a common outcome measure in clinical trials. However, focusing on survival alone may give an inadequate assessment of treatment benefit, by overlooking the quality of life (e.g. morbidity, improved psychological, functional, social, and other factors)4 experienced during any extension in life. The idea of taking quality of life into account when calculating the cost of life-extending treatment was first introduced in a study in 1968.1 It was noted in this research that the quality of life associated with having a kidney transplant was better than that associated with dialysis, by an estimated 25%. Subsequently, the concept of a quality-adjusted life-year (QALY) was introduced. This is a measure embracing both the usefulness or 'utility' of a particular health state as well as the length of life lived under that state.3

Utility is defined by the 'preference' individuals or society express for a particular set of health outcomes.1 The more preferable a specified health outcome, the more utility is associated with it (e.g. two people may attach different preferences with regards to having a broken arm depending on the significance they attach to having full arm movement, which might be heavily influenced by factors such as their occupation). Preferences can be measured using simple rating scales such as a visual analogue scale; 'standard gamble'; 'time-trade-off' (TTO); or the EQ-5D. In standard gamble, the person is asked to indicate whether they would choose to remain in the current state of health for a period of time, or would prefer to gamble on having a treatment which could result in a better, or a worse outcome, than the current state. TTO involves asking the person how many years, living under a chronic state of health, they would be willing to give up in return for living in perfect health but with a reduced life expectancy. EQ-5D (developed by the EuroQol Group) was derived from measuring the preferences of a random sample of around 3,000 adults in the UK using the TTO method. EQ-5D measures mobility, self-care, usual activity, pain/discomfort and anxiety/depression plus 'unconscious' and 'dead' to make a total of 245 health states in all,1 and is NICE's preferred measure of health-related quality of life in adults.

- Cost-utility analysis

Cost-utility analysis (CUA) has many similarities with CEA.1 The incremental health improvement in a CUA is measured in terms of utility (i.e. it considers the individual's or the society's preferences for a health outcome) or quality-of-life outcomes (ideally QALYs). However, unlike CEA, in which the outcomes are single or specific to a particular intervention and unvalued, outcomes in CUA may be single or multiple, are not specific to a particular intervention, and incorporate the notion of value. CUA thereby determines the quality of the health outcome produced or forgone through use of a particular intervention. As CUA has a broad applicability, it is more useful than a CEA to decision-makers1 and therefore, it is the preferred form of economic evaluation for NICE in its technology appraisal process. Both CEA and CUA are techniques that can be used where a decision-maker is considering how to best allocate existing resources, and the assumption is that one of the interventions will be undertaken.

- Cost-benefit analysis

Cost-benefit analysis (CBA) is a method of assessing the net benefit associated with an intervention or interventions. It involves first expressing the benefit of the desired health outcome in monetary terms and then subtracting from this the financial cost of the intervention required to bring about this outcome. This gives the net benefit in monetary terms. CBA is useful in that it has the potential to include costs and benefits indirectly associated with health outcomes, such as time off work taken by family members to care for sick relatives. Benefits in a CBA are valued by people's observed or stated preferences. One method of defining the value is the 'willingness to pay' (WTP) approach, in which the individual or the society is asked how much they would be willing to pay for a treatment to achieve various defined health outcomes. There is, however, a wide variation, and disagreement, on how, and of whose preferences of WTP should be measured.

- Cost-minimization analysis

When the outcomes generated by different interventions are accepted as being broadly equivalent (i.e. have the same effectiveness), and only the comparison of cost is needed, cost-minimization analysis (CMA) can be used to establish which of them provides the best value for money. This could

be the case, for example, when comparing drugs of the same pharmacological class that produce the same clinical outcome.

- Is it cost-effective?

Incremental cost-effectiveness ratio

The most popular method for comparing interventions, using the findings from a CEA or CUA, is to estimate how much additional cost is required for each additional unit of benefit.1 This is the incremental cost-effectiveness ratio (ICER).

- Cost-effectiveness plane

A cost-effectiveness plane is a graphical illustration of cost-effectiveness. Note that comparator (C) is in the origin of the graph and the differences in cost and effectiveness of the new intervention are relative to those of the comparator. A treatment could be said to 'dominate' the alternative, if its effectiveness is higher and its costs lower. That is, the new treatment dominates in quadrant II (because it is more effective and cheaper) and the comparator in quadrant IV (because the new treatment is less effective and more costly). When the new treatment appears in quadrant III, this raises questions as to whether, or to what extent, the current resources can be withdrawn and replaced by the new treatment that is less costly but also less effective. When the new treatment lies in quadrant I, whether its additional effectiveness justifies the additional cost required is usually determined by the treatment's position relative to what is known as the cost-effectiveness threshold (the dotted line). This threshold is the maximum added cost the society is willing to pay per unit of added benefit from the new treatment (i.e. the maximum acceptable ICER). If a new treatment lays above the dotted line in quadrant I, it may therefore be deemed not to be cost-effective, whereas one below the dotted line is cost-effective.

Conclusion

Health economic evaluations are techniques that can be employed to assess benefit from a new treatment relative to its cost. They can also compare new treatments relative to alternative treatments, and are important tools in allocating resources in healthcare. The various types of analysis techniques offer different ways of assessing effectiveness depending on the comparison. Various assumptions made about an intervention contribute to uncertainties or confidence around the findings from health economic evaluations; so results generated must be interpreted with care before making a final decision. The likelihood of the intervention being used may depend on whether its cost-effectiveness falls below a defined threshold. However, in some settings, the threshold is not the sole determinant of whether an intervention is likely to be approved for use.