Interpreting reported health-care benefits

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When reading published reports on the benefits of different treatments or health-care programmes, readers are generally interested in judging the applicability of the results to their own settings. Thus they need to be able to identify what consequences (and costs) were included in the evaluation. This short paper describes the different categories of 'benefit' that may arise from health-care interventions and explains some of the methodological considerations in their measurement. It provides simple advice on avoiding the common pitfalls when interpreting the value offered by a particular treatment or configuration of health services.

Empirical data are increasingly being sought on the benefits of treatments and health services. Intuitively the objectives and the resultant 'benefits' of health care may appear rather obvious — to reduce mortality and morbidity from disease (or sometimes simply to reduce costs while maintaining health gains). However, when reviewing evidence from published research reports, the benefits reported may be restricted to a certain type or a particular viewpoint. Such restrictions have the potential to mislead.

In this short paper we describe the nature of 'benefit' in health-care studies and indicate the main types of benefits which tend to be included in economic evaluations. In addition, we outline the main methods of analysis and approaches to valuation, the aim being to identify common pitfalls when interpreting benefit data. This paper complements our earlier paper explaining health-care costs (Neilson and Davies, 1998). Detailed checklists for assessing economic studies have already been reported elsewhere (Drummond et al, 1996; Jeffereson et al, 1996).

WHAT IS BENEFIT?

We are interested in those services and treatments which deliver more good than harm. Hence an understanding of some basic elements of 'benefit' with respect to health services is necessary if reported studies are to be interpreted correctly.

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The consequences of different health-care interventions or programmes can be identified with respect to their resulting health effects, the utility (or value) of those effects, and their cost consequences. Which of these are included indicates the perspective of the analysis, i.e. who is benefiting from the treatment or programme? The focus could be simply the patient, the patient and the carer, the health service or even society at large.

Effects

The effects of health-care interventions are typically described in terms of changes in morbidity and mortality. Studies which are confined to the consideration of health effects are interested in the impact of treatment in terms of changes in the ability of patients to function physically, socially or emotionally. Sometimes this interest is extended to examining changes in patients' families or carers. Studies focusing on health effects assess efficacy or effectiveness and produce standard measures of effect size such as the number needed to treat (NNT) or relative risk reduction (Davies, 1998).

Utility: quality of life

Not only are the quality of life effects of different treatments and interventions important but the significance or value that patients and their families attach to these effects are also crucial. This valuation of health effects is usually termed their 'utility'. Putting valuations on health changes is highly subjective, practically difficult and sometimes controversial.

Cost consequences

The effects of health care may also be described as changes in resource utilization. For example, an effective screening programme may uncover latent disease never destined to impact on the health service (thereby raising treatment costs), or it may avert potential future costs associated with the treatment of advanced disease. If the perspective of the analysis is the NHS, then these cost consequences may mean a future benefit (savings) or a disbenefit (future cost incurred).

A further (indirect) cost consequence is the impact that treatment may have on patients' ability to perform useful work. A treatment which enables patients to return to work offers benefits to society which should be taken into account. Papers which take a narrow view of benefit may omit some of these potential gains.

Thus the benefits which arise from a treatment or programme may be positive or negative (disbenefits), and are much broader than simply shifts in patients' status (mortality and morbidity). Benefits may encompass the valuations individuals place on health outcomes as well as the cost consequences (both good and bad) of improved treatment.

ESTIMATING BENEFITS

The selection of appropriate units of health-care benefit will depend on which of the three categories of benefit are of interest in a specific evaluation.

Measuring effects

Effects are typically measured in natural units and refer only to an individual's ability to function and not to the significance, preference or value attached to this ability. Common measures of effectiveness relating to mortality and morbidity include life-years gained, deaths prevented, pain-free or symptom-free days, cases successfully diagnosed, complications avoided or cases treated appropriately. Some effects relating to morbidity may be measured using generic health status instruments (measuring physical, social or emotional functioning) and/or disease specific instruments.

In recent years many health status or health-related quality of life scales have been developed. For example the Nottingham Health Profile (NHP) and the Short Form 36 (SF-36, so-called because of its 36 questions) have been used widely in the UK (Bowling, 1995).

Measuring utility

The consequences of alternative treatments or services may also be measured and compared with respect to the (subjective) value of specific levels of health status (utilities). These utilities are derived from the preferences of individuals or society for any particular set of health outcomes. Such preferences are usually expressed on a scale from zero (equivalent to death) to one (equivalent to perfect health).

Techniques for measuring utility include simple rating scales, or more complex approaches such as 'the standard gamble' or 'time trade-off' methods (Torrance, 1986). Each approach generates an adjustment factor with which to increase or decrease the value of time spent in health states. The most common result of utility analyses is quality adjusted life-years (QALYs). Readers should be aware that many issues surrounding QALYs remain unresolved (Neumann et al, 1997). For example, it is well documented that there can exist considerable discrepancies between the patient's valuations and those assigned by health-care providers or the general public (Sprangers and Aaronson, 1992).

Measuring cost consequences

The cost consequences of health-care interventions are typically recorded in terms of changes in resource utilization, for example numbers of procedures performed, equipment costs or amounts of time or space used. Changes in patients' costs might also be included, measured in terms of the quantity of medication purchased or the number of clinic visits (valued in money terms). Valuation of return-to-work or other indirect benefits (such as time saved) may use average wage rates. There are many pitfalls in estimating costs which have been described previously (Neilson and Davies, 1998).

USING MEASURES OF BENEFIT: MAKING COMPARISONS

The health benefits (outputs) from treatments or programmes mean very little on their own without some consideration of the inputs used in their generation. Undertaking economic evaluation studies in which the costs and consequences of alternative treatments and health services are compared simultaneously allows us to address questions of efficiency (i.e. are the interventions worth the costs?).

The benefits of primary interest indicate the type of economic evaluation being performed. Where costs are compared with measures of effect, a cost-effectiveness analysis is being performed; when valuations in the form of utilities are assigned to those effects then this is called a cost-utility analysis. Finally, if the utilities are converted to monetary values — and all the cost-consequences are included — then this is a cost-benefit analysis.

Cost-effectiveness analysis

When the consequences of two health-care interventions (or services) are identical in all relevant respects, our efficiency comparison will be made simply on the basis of cost. However, this type of evaluation — cost-minimization analysis — requires evidence that outcome differences of the alternatives are either non-existent or unimportant.

More usually, there will be differences in the benefits arising from the alternative interventions. In this case, cost-effectiveness analysis compares the benefits of alternative procedures with respect to some common measures of effectiveness. Efficiency questions are then addressed by assessing the results in terms of cost per unit of effect, e.g. the cost per life year gained, cost per case cured, or cost per diagnosis.

Cost-utility analysis

In cost-utility analysis, programme effects are translated into a measure of value (utility) based on preferences for health states. A given set of treatment outcomes are adjusted for the utility attached to those outcomes, thus providing a common denominator for comparison of outcomes and costs in different programmes. To calculate the number of QALYs (the most commonly used utility unit) resulting from a particular intervention, the number of additional years of life gained are combined with a measure of the quality of life in each of these years to obtain a composite index of outcome. For example, two years of life with a utility of 0.5 equates to a single QALY. Comparisons are then based on differences between alternative treatment strategies in the marginal cost per QALY gained.

Cost per QALY 'league tables' have been produced for a host of different treatments and health-care services with the aim of guiding decisions on resource allocation (e.g. hip replacement, breast cancer screening, or heart and kidney transplantation) (Mason et al, 1992). However, a recent review of 86 studies using QALYs found that there was considerable variation in the construction of the QALYs, and that most studies did not adhere to recommended analytical principles (Neuman et al, 1997). Thus studies which generate 'cost-per-QALY' data may not be

directly comparable, having being conducted at different times, using different approaches to measure health status or covering different categories of cost. Other arguments against use of QALYs include allegations that they discriminate against older people, make inappropriate interpersonal comparisons, ignore equity consideration and introduce bias into quality of life scores (Gerard and Mooney, 1993).

Cost-benefit analysis

Cost-benefit analysis extends cost-utility analysis by expressing both the inputs and outputs of treatments and programmes in monetary terms. This requires translating treatment benefits, such as QALYs, medical complications prevented or disability days avoided, into their monetary benefit. It then becomes possible to say whether a particular procedure or programme offers an overall net gain to society in the sense that its total benefits exceed its total costs.

The two main methods of benefit estimation and valuation are the 'human capital approach' and techniques based on individuals' observed or stated preferences (typically 'willingness to pay' surveys) (Sugden and Williams, 1979; Drummond, 1981). The human capital approach measures the benefits of health care (e.g. avoidable morbidity and mortality) in terms of the future flow of income that would otherwise be forgone because of ill health. The willingness to pay approach seeks to establish the value that people attach to health outcomes by asking them how much they (hypothetically) would be prepared to pay to obtain the benefits of care or avoid the costs of illness.

Both approaches are open to criticism. The human capital approach sees people's worth solely in terms of their earning power. This diminishes the worth of the unemployed, the retired, homemakers and children. It also downplays the importance of the non-financial aspects of ill-health such as pain, suffering, loss and grief. However, estimating the value of benefits using willingness to pay surveys is also problematic. The valuations obtained can be sensitive to the way in which questions are asked and in any case tend to be related to respondents' income. Thus converting benefits to a monetary value is certainly no exact science. Therefore a clear understanding of how values were obtained is needed before findings can be judged reasonable.

KEY POINTS

- The outputs of health care may be reported in terms of their health effects, the utility (or value) of those effects, or their cost consequences.
- Cost-effectiveness analysis (CEA), cost-utility analysis (CUA) and cost-benefit analysis (CBA) are methods of economic evaluation used to measure and compare the economic costs and consequences associated with alternative programmes and procedures.
- CEA measures consequences in terms of natural units such as cost per life year saved, cost per case cured, or cost per symptom-free day.
- CUA expresses outcomes as a single utility-based unit of measurement — for example the quality adjusted life year (QALY).
- In CBA monetary values are placed on both the inputs (costs) and the outcomes (benefits) of health care to give an overall estimation of net monetary benefit.
- Ideally, cost-effectiveness studies should be built-in alongside clinical trials, or should draw on published data. In the absence of data, any assumptions must be made explicit.
- Sensitivity analyses should be applied when there is uncertainty about the effectiveness (and costs) of different procedures to investigate the extent to which results are sensitive to alternative assumptions about key variables.
- The discounting of benefits is controversial although most economic evaluations do chose to do this.

Although studies generally state which economic evaluation approach is being used it is important to check whether this is indeed the case. A recent review of 95 studies labelled as cost-benefit evaluations and appraised against standard definitions of this technique revealed that 53% were actually only partial evaluations because health gains were not properly evaluated (Zarnke et al, 1997).

BENEFIT NOW OR BENEFIT LATER?

It is part of human nature to want our benefits now and our costs deferred. Because of this, some would argue that both costs and benefits obtained some way into the future are worth less than they would be if obtained in the present. This reduction in value into the future is called 'discounting'.

The traditional view has been that future benefits should be discounted in the same way as future costs, although arguments have been advanced both for and against such a move (Cairns, 1992; Parsonage and Neuberger, 1992). What is of importance to the reader, however, is not whether we should or should not discount benefits, but what are the consequences for the main study findings if one view is adopted over another. Discounting benefits means that procedures with long-lasting effects such as neonatal care, maternity services, health prevention and promotion activities — which lead to benefits over the recipient's entire future lifetime — receive lower priority. Not discounting benefits would change the relative cost-effectiveness of different procedures and make projects with long lasting effects relatively more cost-effective.

SENSITIVITY ANALYSIS

Users of economic evaluations will be interested to know how robust the initial results are to variations in the values of the key estimates and assumptions used. For example, the size of any treatment effect may vary between patient groups as may the costs incurred. Reporting such 'sensitivity analyses' enables readers to consider to what extent the authors have

allowed for uncertainties inherent in their analysis. This is not always done in published work: two recent methodological reviews of published cost-effectiveness and cost-benefit analyses found that many evaluations failed to make underlying assumptions explicit and failed to test assumptions with sensitivity analyses (Udvarhelyi et al, 1992; Briggs and Sculpher, 1995).

CONCLUSIONS

Studies which report on the benefits of treatments and services require the reader to be familiar with some of the important elements of appropriate evaluation techniques. Many difficulties arise in measuring, let alone valuing, the benefits arising from health-care interventions. Thus it is impossible to judge the headline findings from economic analyses without a detailed assessment of all the individual judgments or estimates made along the way.

In addition, it is important to check the data sources used for the analysis — data may be suspect for either inputs or outputs, and the conclusions of any analysis are only as good as the underlying studies used to estimate the base data. Even so, because of the uncertainty associated with these issues, thorough sensitivity analyses exploring the robustness of the conclusions are an essential factor in building confidence in the findings. HM

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