

Trent Focus for
Research
and Development in
Primary Health Care

Health Economic
Evaluation

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HEALTH ECONOMIC EVALUATION

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Introduction

Primary health care teams play a pivotal role in the health care system. As gatekeepers to the health service their decisions have significant impact on people's health and on health service resource use. This role is likely to become even more vital as they take on even more decision-making powers which have implications for a larger range of health services and a larger share of health resources. In simple terms, primary care professionals face recurring questions about: who should do what to whom, with what health care resources, and with what relation to other health services? The answers to these questions depend on estimates of the relative merit or value of alternative courses of action. Health economic evaluation uses a range of strategies and tactics whereby these estimates of relative value can be ascertained and interpreted. These tools are needed, as it is no longer acceptable for decision-makers to simply choose strategies that work, or even those that work best, but rather those that work best subject to the best use of resources. Decision-making based on evidence of economic efficiency will ultimately allow more things to be achieved with the same level of resources.

This pack provides a way into the sometimes complex world of health economics. It is a practical guide on what health economics can do for the primary care professional. For those readers only hoping to gain a brief overview of what Health Economics is, then Chapters 1-4 are recommended reading.

Learning objectives

Having successfully completed this pack, you will be able to:

- ◆ understand the role of economic evaluation in providing information for decision-makers
- ◆ understand how these evaluations should be constructed
- ◆ distinguish between the different types of economic evaluation and when each may be appropriate
- ◆ discuss the issues and problems involved

The overall aim of this pack is twofold: to enable the reader to better understand, critique and interpret published economic evaluations in the medical literature; but also to provide primary care professionals with the tools needed to begin to carry out their own economic evaluations.

The Health Economy

The rapid growth in expenditure on health care is an international problem fuelled by demographic changes, technological advance and changing expectations. The UK currently invests around £50 billion a year in the National Health Service, which constitutes approximately 7% of the nations annual output (Gross Domestic Product). Interestingly, this is comparatively low relative to most other Organisation for Economic Co-operation & Development (OECD) nations and especially the United States, which invests twice this level at nearly 15% of GDP. In all developed countries, however, one thing is clear: the ‘health economy’ is very large and hugely important. Decision-makers in health care face an awful lot of difficult choices, the subject of economics aims to help with some of these.

The Economics Perspective

What is Economics?

“The study of how men and society end up choosing to employ scarce resources that could have alternative uses” (Samuelson)¹

Simply, economics is about allocating scarce resources. Any introductory economics textbook will have a quote similar to the one above, which contains three elements fundamental to understanding the economic perspective: choosing, scarce resources and alternative uses.

First, ‘choosing’ or decision-making is what the discipline of economics strives to analyse and ultimately assist with. Indeed, economics has been labelled the ‘science of choosing’. It aims to provide a framework for choice so that the full implications of all choices are clearly identified before they are made.

Second, scarcity is known as the economic problem. Scarcity exists since needs, wants, demands or desires will always be greater than resources available to meet them. This is a fundamental starting point for the economic perspective.

Third, economists differ to say, accountants in the way that they conceptualise cost. They think about the possible alternative use of any resources, the notion that economists call ‘opportunity cost’. Opportunity cost is a key concept underpinning the economic perspective.

The real cost of doing one thing is not actually the pounds you spend but the opportunity of doing something else with this money. Yes, it is important to know the financial implications of a certain action you choose to do but it is the benefits that you could have derived from what you did not choose to do that is the real cost of this action. Now, choosing to use resources one way will always mean giving up the chance to use them in other desirable ways. So, the question that economists ask is:

¹ Samuelson P. (1980) Economics. McGraw-Hill

“Are the benefits from what is ‘chosen’ greater than what is ‘forgone’?” Thus, opportunity cost can be defined as the benefits given up in the best alternative use of the resources.

Economics of Health

"What a buyer wants to know is the difference between his state of well-being with and without the commodity being considered. For ordinary goods, the buyer has little difficulty in evaluating the counter-factual - that is what the situation will be if the good is not obtained. Not so for the bulk of health care...the noteworthy point is not simply that it is difficult for the consumer to judge quality before the purchase...but that it is difficult even after..."
(Weisbrod BA²)

So why is this economic perspective useful in the context of health care? Health economics examines the problem of scarcity as it arises with respect to health and health care. It examines how we as individuals and societies confront the fact that while the resources available to us are limited the alternative uses for these resources are unlimited. Thus health economists are interested in some very important questions. How is health produced? What role does health care play in its production? What is the value of health? How do we go about measuring health status? What influences demand for health and health care? What influences the supply of health care? How can equilibrium between demand and supply be achieved? The discipline of health economics is the study of these questions and the answers to them that individuals and societies have put forward.

So what do health economist's actually do? Broadly, health economists are engaged in activities under three categories: the study of the principles of health economics; health technology assessment; and health systems assessment. The principles of health economics consider supply and demand issues and how the two might interact given that the standard market solution generally fails due to problems such as adverse selection, moral hazard, asymmetric information and supplier induced demand³. However, much of a health economist's time is spent on assessment either of specific health technologies or more widely health systems.

² In Greenberg W (Ed) 1978. *Competition in the health care sector: proceedings of a conference.*

³ *Adverse selection: an event in healthcare whereby one party decides not to reveal the full extent of their risk profile to the other party (i.e. insurance model). Moral hazard: arises where the attitudes and behaviour of a person or organisation change once they are covered for potential costs or losses (e.g. healthcare consumption may be higher when insured.) Asymmetric information: doctors have more knowledge and information about medicine than patients/consumers, the individual may not be the best judge of his/her own interests, the doctor acts as an agent of the patients demand.*

Scarcity demands that choices must be made as to what health care should be provided, how it should be provided, in what quantities and how it might be distributed. Economic evaluation is the area of health economics used to help address these issues.

Economic Evaluation

"the pursuit of efficient practice is not merely about reducing costs. If it were, the most efficient procedure would be to do nothing, as that pushes costs to zero." (Professor Alan Maynard⁴)

Economic evaluation of health care programmes aims to aid decision-makers with their difficult choices in allocating health care resources, setting priorities and moulding health policy. But it might be argued that this is only an intermediate objective. The real purpose of doing economic evaluation is to improve efficiency: the way inputs (money, labour, capital etc.) can be converted into outputs (saving life, health gain, improving quality of life, etc.)

The choice of what health care to provide is about what economists call allocative efficiency. This means that we strive for the maximisation of benefits (however we decide to measure this) subject to given available resources. So, from a fixed resource we aim to get as much out of a range of health care programmes as possible. This will mean we will need to compare very different interventions, say health promotion advice to quit smoking versus prescribing Relenza versus a procedure on an ingrown toenail. Thus allocative efficiency is about finding the optimal mix of services that deliver the maximum possible benefit in total. Resources will be directed to interventions that are relatively good (i.e. efficient) at converting inputs into health benefits and away from those that require larger input for relatively low health gain. This approach may of course be constrained by certain equity considerations, to ensure that certain groups do receive health care.

The choice of *how* to provide health care is about what economists call technical efficiency. This means that we might strive for minimum input for a given output. For example, if we have decided that performing tonsillectomies on children is worthwhile, part of an allocatively efficient allocation of resources, then we may need to examine the efficiency of how we do this. So, if the output we wish to achieve is to successfully remove a child's tonsils then we might choose between, say, a day case procedure or an inpatient stay. This is an issue of technical efficiency since the output or 'outcome' is fixed but the inputs will differ depending on which policy we adopt. The day case approach may perhaps require more intensive staff input and more follow-up outpatient visits. If this were the case then inpatient tonsillectomy may be the more technically efficient strategy.

⁴ In Jefferson T et al. (2000) Elementary economic evaluation. BMJ books: London, p.3

Thus with any given health care programme an economic evaluation is aiming to make explicit the total resources consumed specifically by that programme (i.e. attributable to it) and the total benefit generated specifically by that programme.

Drummond et al (1997) defines economic evaluation as “the comparative analysis of alternative courses of action in terms of both their costs and consequences.” It differs from other forms of analysis because it considers both costs and consequences and is comparative.

Evaluation needs to be comparative as an intervention can only be labelled as good or bad relative to some benchmark or alternative even if this alternative is a ‘do nothing’ strategy. If an evaluation is not comparative and does not consider both costs and consequences, then it is only a partial evaluation. It is a description of either just the costs or just the benefits of one intervention in isolation. This is most uninformative since it is one-dimensional and without a context by which to judge relative performance (efficiency). If both costs and consequences are considered but no comparator is provided, then the study is again only a partial evaluation, described as a cost-outcome study. It lacks context and is of limited use. If alternatives are compared but only in terms of costs or benefits and not both then again the study only provides a partial evaluation and can be labelled an effectiveness study or a cost analysis. It would be comparative but only across one-dimension. Hence, an economic approach can be considered a full evaluation technique. (See figure 1)

Figure 1 - Defining An Economic Evaluation

(Reproduced from Drummond et al., 1997)

| <i>Health Evaluations</i> | | | |
|--|-----|---|------------------------|
| Are both costs & consequences of the alternatives examined ? | | | |
| | | NO | YES |
| Is there comparison of two or more alternatives ? | NO | outputs only | costs only |
| | | 1A PARTIAL EVALUATION outcome description | 1B cost description |
| | YES | 3A PARTIAL EVALUATION Efficacy or effectiveness evaluation | 3B Cost analysis |

*Note: c-e-a = cost effectiveness analysis
 c-u-a = cost utility analysis
 c-b-a = cost benefit analysis
 See page 10 for explanation*

Costs and Consequences

"What is a cynic? A man who knows the price of everything, and the value of nothing" (Oscar Wilde⁵)

Costs can be defined in many ways (see figure 2) but generally can be considered as direct, indirect and intangible. Direct costs are those immediately associated with an intervention such as staff time, consumables etc. Indirect costs might include a patients work loss due to treatment. Intangible costs may be things like pain, anxiety, quality etc. All types of economic evaluation deal with costs in the same way or at least in the same units (i.e. monetary).

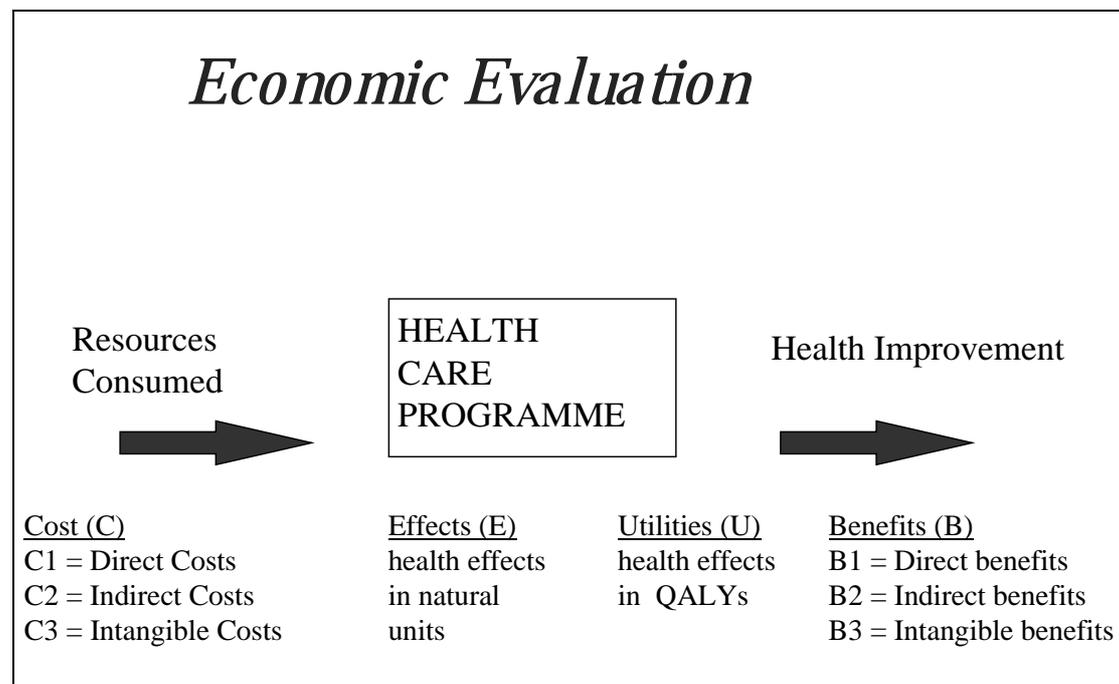
Benefits, however, can be analysed in three different ways reflecting the different types of economic analysis used in evaluation. First, benefits can be examined in terms of the immediate (direct) effects on health. These are usually clinically defined units appropriate to the area of study, such as 'lives saved', 'reduction in tumour size', 'change in blood pressure' etc. Second, benefits from an intervention can be considered in more generic terms such as the impact on general well-being/happiness/ satisfaction, these are more generally labelled as 'utilities'. The utility of an intervention to an individual is its benefit. Measures such as the quality adjusted

⁵ In: Lady Windermere's Fan.

life year (QALY) are used to quantify this. Third, benefits might be considered in the same terms as costs, which means that benefits must be valued in monetary terms by some means.

Figure 2 - Evaluating Costs and Consequences

(Reproduced from Drummond et al., 1997)



Whatever the approach the same three-stage process for the assessment of all costs and benefits can be applied. All relevant cost and benefit variables must be i) identified, ii) quantified and iii) valued.

At the start of an evaluation it must be determined which costs and benefits are sufficiently important to merit inclusion in the study. This should be separate from the measurement stage so as to avoid the study being entirely data driven (i.e. the more intangible consequences of an intervention might be considered equally important). The identification of relevant benefits and costs will define the variables in the study. These can be broadly classified into changes in resource use, changes in productive output and changes in health state.

The next stage is to measure changes in these variables brought about by the intervention in question. Often it is important that this is done before valuation, as it is necessary to know the magnitude of gains or losses before values can be attached. Presenting variables in terms of 'natural' quantities or frequencies (i.e. hours worked or clinical units) can also be very useful in terms of generalisability. Others can use these data and apply values relevant to their own setting (i.e. different cost structures or health values).

Quantifying changes in resource use is in terms of land, labour, capital or consumables. Labour, the predominant element of most health care, is often expressed in units of time that can later be valued in some way. The quantification of raw materials e.g. amount of drugs, dressings, appliances, is usually straightforward as these are counted and recorded by routine data systems. Labour and consumables are less problematic as they can normally be attributed specifically to a study intervention. Quantifying a specific intervention share of 'shared resources' such as capital stock and land (equipment, overheads, buildings) is more of a thorny issue and there are various accountancy techniques to resolve this.

There are several issues to consider in the assessment of costs and benefits. Externality costs and/or benefits may arise since interventions do not just affect the patient receiving care. For example, if I receive treatment for a contagious disease you will benefit as well as me, since your chances of contracting the disease will be reduced. Any evaluation needs to account for this.

The differential timing of costs and benefits must also be considered in an evaluation. The effects of health treatments do not always occur at the same point in time. Costs may be incurred today but the benefit may not arrive until next year (i.e. preventative treatments, health promotion), part of this future benefit might be that future costs will be avoided. £100 spent today may not have the same value as £100 spent next year because of inflation, interest on savings and not least a positive rate of time preference. People may just prefer to have £100 in their pocket today rather than £100 in a week or a month or a year, because it offers them more choices. This can be incorporated into economic evaluation by the notion of discounting future costs and benefits to their present day value. A simple formula can be applied to do this for any chosen discount rate, normally within the range of 0-10%.

Material covered so far in this pack has been very much at a conceptual level. Before we move onto some more practical applications the following revision questions may be useful.

Self-assessment: questions

Multiple choice questions

- 1) The statement 'There is no such thing as a free lunch' relates to the application of:
 - a) the concept of technical efficiency
 - b) the concept of allocative efficiency
 - c) the concept of opportunity cost
 - d) the concept of the margin

- 2) The technique of discounting in an economic evaluation is to adjust for:
 - a) the reduced cost of buying in bulk
 - b) the exclusion of certain variables
 - c) the effect of inflation on costs
 - d) the differential timing of costs and benefits

- 3) Allocative efficiency is about:
 - a) the best way to achieve a given outcome
 - b) maximising total benefits within a given budget
 - c) being aware that everyone is allocated a fair share
 - d) spending as much of an allocated budget as possible

Answers given at the end of this Resource Pack

Types of Economic Evaluation

The different ways of looking at benefits combined with cost analysis represent the different techniques of economic evaluation: cost effectiveness analysis (CEA), cost utility (CUA) and cost benefit analysis (CBA). When to use each of the above techniques will depend of the nature of the question to be addressed, which may be a choice between alternative clinical strategies for a condition; timing of an intervention; settings for care; types and skill-mix of personnel providing care; programmes for different conditions; scale or size of a programme; or other ways to improve health.

Cost-Effectiveness Analysis

CEA is concerned with technical efficiency issues, such as: what is the best way of achieving a given goal or what is the best way of spending a given budget. Comparisons can be made between different health programmes in terms of their cost effectiveness ratios: cost per unit of effect. Under CEA effects are measured in terms of the most appropriate uni-dimensional natural unit. So, if the question to be addressed was: what is the best way of treating renal failure? Then the most appropriate ratio with which to compare programmes might be 'cost per life saved'. Similarly, if we wanted to compare the cost-effectiveness of programmes of screening for Down's syndrome the most appropriate ratio might be 'cost per Down's syndrome foetus detected'. In deciding whether long-term care for the elderly should be provided in nursing homes or the community the 'cost per disability day avoided' might be the most appropriate measure.

The advantage of the CEA approach is that it is relatively straightforward to carry out and is often sufficient for addressing many questions in health care. However, it is not comprehensive. The outcome is uni-dimensional under this analysis but often health programmes generate multiple outcomes. For example in Down's syndrome screening, foetuses detected is one outcome, but miscarriages avoided might be another very relevant outcome measure, especially if, say, blood testing is being compared to amniocentesis. But this cannot be incorporated into this form of analysis. So CEA not only assumes that the outcome of the health programme is worthwhile per se but also that it is the most appropriate measure. A further problem with CEA is comparability between very different health programmes. Cost per foetus detected may be a useful way to compare the efficiency of blood testing versus amniocentesis but how would these be compared to, say, drugs aimed at reducing cholesterol. Health programmes with different aims cannot be compared with one another using CEA: cost per unit reduction in cholesterol cannot meaningfully be compared with foetuses detected. Hence CEA is useful when comparing programmes within like areas, where common 'currencies' can be used.

The following case study shows how cost-effectiveness analysis may be used in practice.

Case Study 1

A Cost-effectiveness study - Exercise therapy for Knee-Pain

Background. Knee pain is common in the general population and a major cause of morbidity. Much of this is attributable to osteoarthritis. The cost of musculoskeletal disease is high (estimated at 2.5% of GNP in United States, 1992) and osteoarthritis is the commonest joint disease. In addition to costs arising directly from symptoms and treatment of osteoarthritis, patients with osteoarthritis have significantly higher medical costs for a range of other conditions (respiratory, cardiovascular, gastrointestinal, neurological, psychiatric conditions and general medical care). Thus the economic burden of this disease is high.

Treatments. The two main palliative treatments for knee pain are exercise or non-steroidal anti-inflammatory drugs (NSAIDs). NSAIDs are commonly used in the treatment of osteoarthritis but are costly and can cause gastrointestinal problems. Exercise may be a more favourable alternative since quadriceps muscle strength is known to be reduced in osteoarthritis. Since this weakness is associated with disability it is pertinent to examine the costs and consequences of muscle strengthening regimes.

Study Objective. To evaluate the cost-effectiveness of regular home exercises in reducing the burden of knee pain in the community compared with placebo drug.

Study Methods. An economic evaluation was conducted prospectively alongside a randomised controlled trial. Cost data were collected from GP case notes and by patient questionnaire. Outcomes were collected by the clinical trial. Economic analysis was in the form of a cost-effectiveness study.

Outcomes. The principle outcome measure was change in knee pain at two years measured using the Western Ontario MacMaster's Universities Osteoarthritis Index (WOMAC) knee specific assessment questionnaire.

Costs. Three main categories of cost were included in this analysis: Direct treatment costs incurred by the treatment programme. Knee-pain related medical costs (hospital and community). Knee-pain related costs to patients and family related to accessing health services.

Results. The overall cost of achieving a clinically significant reduction in pain (greater than 50% on the WOMAC scale) after 2 years was compared for each intervention. For the exercise group this was £1,024 and for the placebo group this was £129. Thus, due to relatively high training costs and low effectiveness, the exercise intervention was found to be less cost-effective than a placebo drug.

Comment.

This study addresses a technical efficiency issue. What is the best way of achieving a given clinical outcome? A cost effectiveness analysis is therefore the most appropriate study design. Comparison is confined to interventions within the same disease and condition area so a single outcome measure is sufficient. However, given that knee-pain may impact on quality of life more generally a uni-dimensional outcome such as WOMAC pain score may not be the most relevant measure. Multi-dimensional outcome measures may in fact produce a different economic result.

Ref. Doherty M et al. A community based randomised intervention study examining the effects of exercise on knee pain and its associated disability. Final report to the Department of Health. April 1999.

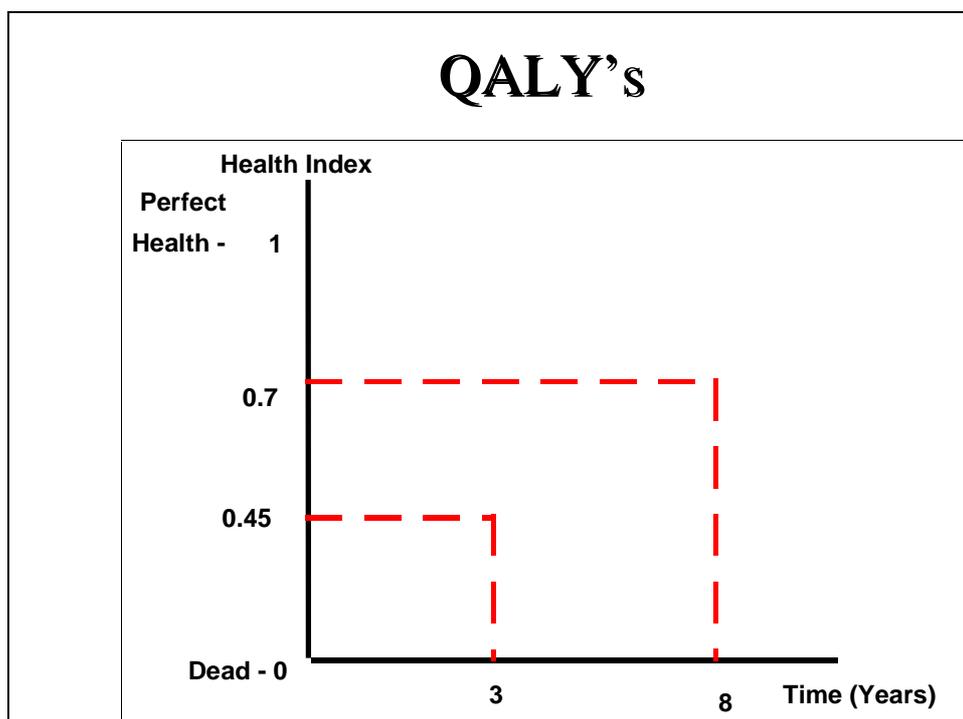
Cost-Utility Analysis

CUA is concerned with technical efficiency and allocative efficiency (within the health care sector). It can be thought of as a sophisticated form of CEA, since it also makes comparisons between health programmes in terms of cost-effect ratios. However, CUA differs in the way it considers effects. These are multidimensional under this form of analysis. CUA tends to be used when quality of life is an important factor involved in the health programmes being evaluated. This is because CUA combines life years (quantity of life) gained as a result of a health programme with some judgement on the quality of those life years. It is this judgement element that is labelled utility. Utility is simply a measure of preference, where values can be assigned to different states of health (relevant to the programme) that represent individual preferences. This is normally done by assigning values between 1.0 and 0.0, where 1.0 is the best imaginable state of health (completely healthy) and 0.0 is the worst imaginable (perhaps death). States of health may be described using many different instruments (SF-36, Nottingham Health Profile, Sickness Impact Profile, EuroQol EQ-5D), which provide a profile of scores in different health domains. EuroQol EQ-5D for example simplifies health into just five domains (figure 3): mobility, self-care, usual activities, pain/discomfort and anxiety/depression. Each domain is given a score from 1 to 3, so the health profile would read 11111 for the best scores in all domains and 33333 for the worst. EuroQol EQ-5D has 243 possible health profiles, all of which have been assigned a utility value by general population surveys. (For further details see Kind, 1996)

Figure 3 - EuroQol EQ-5D

| | |
|---|--------------------------|
| Mobility | |
| I have no problems in walking about | <input type="checkbox"/> |
| I have some problems in walking about | <input type="checkbox"/> |
| I am confined to bed | <input type="checkbox"/> |
| Self-Care | |
| I have no problems with self-care | <input type="checkbox"/> |
| I have some problems washing or dressing myself | <input type="checkbox"/> |
| I am unable to wash or dress myself | <input type="checkbox"/> |
| Usual Activities | |
| I have no problems with performing my usual activities (e.g. work, study, housework, family or leisure activities) | <input type="checkbox"/> |
| I have some problems with performing my usual activities | <input type="checkbox"/> |
| I am unable to perform my usual activities | <input type="checkbox"/> |
| Pain/Discomfort | |
| I have no pain or discomfort | <input type="checkbox"/> |
| I have moderate pain or discomfort | <input type="checkbox"/> |
| I have extreme pain or discomfort | <input type="checkbox"/> |
| Anxiety/Depression | |
| I am not anxious or depressed | <input type="checkbox"/> |
| I am moderately anxious or depressed | <input type="checkbox"/> |
| I am extremely anxious or depressed | <input type="checkbox"/> |

These utility values are then combined with survival data to derive quality-adjusted life years (QALYs) for different health programmes. For example, assume that a patient who receives no treatment has a life expectancy of 3 years and their quality of life has a value 0.45. Now, if this patient receives a certain intervention then it is expected that life expectancy will be 8 years and the quality of those years will have a value of 0.70. The multidimensional gain from the intervention can then be summarised. With no treatment 1.35 QALYs (3×0.45) are produced, with treatment 5.60 QALYs (8×0.70) are produced, thus the gain is 4.25 quality adjusted life-years. Figure 4 shows this graphically.

Figure 4 – The Quality Adjusted Life Year (QALY)**Table 1 – Cost per QALY gained ‘league table’**

| Cost per QALY League Tables | |
|--------------------------------------|-----------------------------|
| <i>Intervention</i> | <i>Cost per QALY</i> |
| <i>GP anti-smoking advice</i> | <i>£250</i> |
| <i>Pacemaker</i> | <i>£700</i> |
| <i>Hip replacement</i> | <i>£750</i> |
| <i>CABG for MVD</i> | <i>£1,040</i> |
| <i>Hospital haemodialysis</i> | <i>£14,000</i> |
| <i>Beta interferon for MS</i> | <i>£285,000</i> |

This approach of using utility is not restricted to similar clinical areas but can be used to compare very different health programmes in the same terms. As a result ‘cost per QALY gained’ league tables are often produced to compare the relative efficiency with which different interventions can turn resources invested into QALYs gained. Table 1 shows that with this approach it is possible to compare surgical, medical,

pharmaceutical and health promotion interventions with each other. Comparability then is the key advantage of this type of economic evaluation. For a decision-maker faced with allocating scarce resources between competing claims, CUA can potentially be very informative. Table 2 lists some of the advantages and disadvantages of this approach. However, the key problem with CUA is the difficulty of deriving health benefits. Can a state of health in fact be collapsed into a single value? If it can then whose values should be considered in these analyses? For these reasons CUA remains a relatively little used form of economic evaluation.

Table 2 - Advantages and disadvantages of Cost per QALY gained 'league tables'

| |
|--|
| <p>Pros</p> <ul style="list-style-type: none"> • <i>reveals opportunity cost</i> • <i>common currency</i> • <i>comparison across diseases</i> • <i>considers length and quality of life</i> • <i>investment type problem - "best returns"</i> • <i>underlying principle - buy "cheap" QALYs not "expensive" QALYs</i> <p>Cons</p> <ul style="list-style-type: none"> • <i>What of equity?</i> • <i>a QALY is a QALY is a QALY, or is it ?</i> • <i>what of equality of access?</i> • <i>only health service costs</i> • <i>What of other health benefits?</i> • <i>patient information / reassurance</i> • <i>Comparability of C-U-A studies</i> • <i>lack of them!</i> • <i>apply locally?</i> |
|--|

The following case study shows how cost-utility analysis may be used in practice.

Case Study 2

A Cost-utility study - Interferon Beta for Multiple Sclerosis

Background. Clinical trials have established that interferon beta preparations do have some effect in reducing MS disease activity. This has been reported in terms of reduction in number of relapses or lesion size identified by MRI. However, little is known about the impact on quality of life or how cost-effectively this can be generated using this intervention.

Study Objective. To identify to what extent interferon beta generates quality of life (QOL) gains. To measure and value QOL gains. To assess the net costs to the health service and society associated with interferon beta. To compare net costs and QOL gains in a cost-utility model.

Study Methods. Data were collected from existing trials of interferon beta and from information on the natural history of MS. New data were collected on QOL and costs from a group of MS patients. A sub-group was used for utility measurement (the valuation of different health states).

Outcome. The key outcome measure was cost per quality-adjusted life year (QALY).

Results. Using current clinical data on the effectiveness of interferon beta the best estimate in terms of a cost-utility ratio was £809,000 per QALY gained. Allowing for a possible impact on disease progression over different time periods produced cost-utility ratios in the range £228,300 - £328,300. Thus interferon beta does produce gains in QOL but these are occasional and short-term and can only be achieved with a very large additional cost.

Comment.

This study gathers data to supplement existing information about this drug and constructs a model to aid decision-making. CUA is the appropriate study design since change in QOL is an essential outcome of this intervention. Also for comparability purposes CUA presents the decision-maker with a common currency across different disease groups.

Ref. Parkin D, McNamee P, Jacoby A, Miller P, Thomas S, Bates D. A cost-utility analysis of interferon beta for multiple sclerosis. Health Technology Assessment, 1998; Vol.2: no.4

Cost-Benefit Analysis

CBA is concerned with allocative efficiency. Under this form of economic evaluation costs and benefits are measured in commensurate units (normally money). Whilst the other forms of economic evaluation deal with relative efficiencies, CBA can be used to evaluate health programmes in a more absolute way. So we can ask is intervention X worthwhile per se? Are the benefits greater or less than the costs? CBA can reveal the net economic impact of an activity: gain or loss. Only activities that generate a net economic gain might then be considered further by comparing the magnitude of the gain under different activities.

CBA can be used to consider allocative efficiency in its widest sense because once benefits have been converted into monetary terms then the net economic impact of very different activities can be compared. The gain to society from say, building a new bridge might be compared with prescribing a new pharmaceutical. Resources might be reallocated based on the results of CBA until the point when any further reallocation of resources cannot make anyone better off without making at least someone else worse off. This is the point known as 'Pareto efficiency', named after a famous economist Vilfredo Pareto.

The main problem with the CBA approach in health care is very obvious: how do we measure or 'convert' benefits from health programmes into monetary values? This is a very difficult issue and many health economists would still argue that it is futile to do so. There are, however, two main techniques for the monetary valuation of benefits: the 'human capital' and the 'willingness to pay' approaches. With the human capital approach the benefit of a health programme is measured by how it helps the patient return to, or increase, their productive output. Productive output can be easily valued using actual or proxy wage rates. Clearly this approach will not always be appropriate especially in the case of children or the elderly. The willingness to pay approach assumes that the utility an individual gains from an intervention is valued by the maximum amount they would be willing to pay for it (out of their own pocket!). The willingness to pay technique simply presents people with hypothetical scenarios where they must decide the maximum amount of their own income they would give up in order to receive a benefit or avoid a cost. Various research methods have been developed to illicit from individuals their monetary valuation of health benefit. Hypothetical questions may be asked in a closed or open-ended manner. This has proved to be a very successful research area and is developing rapidly. However, it is still difficult to get away from the fact that inevitably willingness to pay is a function of ability to pay and results may be more a reflection of wealth than valuation of benefit. (For further details of the willingness to pay method see Diener, 1998)

The following case study shows how cost-benefit analysis may be used in practice.

Case Study 3

A Cost-benefit study - Occupational Health Services

Background.

Most large organisations choose to provide an occupational health service (OHS) beyond that which is required by law. Whilst the input costs (labour, capital, etc) of OHS are very clearly identifiable the outputs are not always directly observable since benefits may be multidimensional and sometimes inherently intangible. Consequently it is unclear whether the benefits of these activities outweigh their costs. In an environment of competition for resources lack of evidence on cost-effectiveness is likely to be regarded the same as activities demonstrated not to be cost-effective, whereas those activities that can demonstrate cost-effectiveness will be supported

Study Objective. To quantify the value added by OHS for a specific organisation.

Study Methods.

OHS was conceptualised as a form of insurance policy, which individual managers chose to purchase at different levels of cover. Under this model OHS is purchased in order to reduce the risk and impact of negative events whose cost, timing and frequency is uncertain. The contingent valuation methodology was used to elicit monetary valuations of benefits from these managers. It is based on "stated" rather than "revealed" preferences. In a market, preferences are revealed by individual's actions the survey-based contingent valuation methodology (CVM) requires individuals to state values for a particular good or service. By asking for stated preferences, CVM allows for a monetary valuation of a particular 'project's' benefits. Contingent valuation has two types of question to elicit values, 'Willingness-to-pay' (WTP) and 'Willingness-to-accept' (WTA) questions. OH cost data are then compared with benefit valuations generated by the contingent valuation survey in order to construct a cost-benefit analysis.

Results.

The maximum amount respondents are 'willing to pay (WTP)' for the benefits provided by OHS is £300 per employee per year (median value). The minimum amount respondents are 'willing to accept (WTA)' as compensation for a withdrawal of OHS is £400 per employee per year (median value). The aspect of OHS valued most highly is the ability to enhance workplace safety. The aspect valued least is the possible impact on reducing medico-legal costs. Cost-benefit analysis shows that OHS generates a positive value added range. Sensitivity analysis shows that WTP and WTA values would have to be considerably lower before the overall economic result is reversed.

Comment.

In the absence of data on the benefits of OHS, WTP and WTA techniques provided a means of quantifying and moreover valuing the multidimensional consequences of engaging in this activity. These results were then used in a simple cost-benefit equation, to measure net economic impact.

Different types of economic evaluation are required depending on what is being evaluated, what the setting is, what the objectives are, who the evaluation is for and what the study perspective might be. Tables 3-7 provide a summary of the different economic analysis tools available to the decision-maker and how they may help.

Table 3 - Tools to aid decision-making

- ***Cost minimisation analysis***
- ***Cost effectiveness analysis***
- ***Cost utility analysis***
- ***Cost benefit analysis***

Table 4 - Cost Effectiveness Analysis

- ***consequences measured in the most appropriate natural or physical unit***
- ***eg. 'years of life gained' or 'mmHg' etc.***
- ***depends on objectives of intervention***
- ***comparability?***
- ***not a question of 'should we do this intervention?' more 'how much?'***

Table 5 - Cost Benefit Analysis

- ***values consequences in terms of money (same unit as costs)***
- ***interventions can be valued absolutely***
- ***(do the benefits outweigh the costs?)***
- ***potentially broadest form of evaluation***
- ***comparable by net gain***
- ***BUT - measurement problems***

Table 6 - Cost Utility Analysis

consequences are measured in time units adjusted by health utility weights

- ***states of health are valued relative to one another***
- ***includes both quantity and quality of life***
- ***morbidity as well as mortality***
- ***common currency ('utility') enables comparison of alternatives***

Table 7 - How do these aid decision-making?

- ***c-m-a tells you the most technically efficient way to achieve an objective (given limitations)***
- ***c-e-a tells you the cost per unit of outcome, without valuing different outcomes (O.K. within like areas)***
- ***c-b-a tells you whether or not an intervention is worthwhile (measurement?)***
- ***c-u-a can provide a framework for priority setting - QALY league table***

The following self-assessment questions may be useful to test your understanding of the different types of economic evaluation and when they might be used. Answers are given at the back of this pack.

Self-assessment questions

Which technique?

Consider each of the following examples and decide which economic evaluation technique it would be appropriate to use. For each example, also state what your measure of effectiveness/benefit would be and why. If you do not think there is enough information to decide, then say so and why you think this is the case.

- a) A decision has been made to introduce a breast cancer-screening programme and to spend a maximum of £500,000 on it. The purpose of the programme is stated to be “to extend the lives of as many women as possible within the budget constraint”.
- b) A decision has been made to introduce a breast cancer-screening programme and to spend a maximum of £800,000 on it.
- c) A decision has to be made on whether or not to introduce a breast cancer-screening programme.
- d) A decision has to be made on which women to screen in a breast cancer-screening programme.
- e) A decision has to be made on how often to screen women for breast cancer.
- f) The objectives of an exercise in priority setting across different technologies – renal failure, coronary artery bypass grafting, hip replacement, etc. – is simply to maximise health from the health service budget.
- g) The objectives of an exercise in priority setting across different technologies – renal failure, coronary artery bypass grafting, hip replacement, etc. – is simply to maximise benefit from the health service budget.

Economic Evaluation: Actually Doing It!

Discussion of the different types of economic evaluation has thus far been at a conceptual level. Economic evaluation however, is an applied subject. Perhaps the best way to understand these tools is with some illustrative data. Consider the example in table 8. Here there are three projects to be compared (A,B,C). Using the "identify-measure-value" process researchers have gathered a range of data which is presented in the table.

Table 8 - A note on comparing projects

| | Project A (current practice) | Project B | Project C |
|--|------------------------------------|------------------|---------------------|
| Effect | 10 | 11 | 30 |
| Cost (£000's) | £50 | £60 | £200 |
| Monetary valuation of effect (£000's) | £54 | £59 | £162 |
| CBA - net benefit (£000's) | £4 | -£1 | -£38 |
| Average cost-effectiveness ratio (Cost / Effect) £000's) | £5 | £5.5 | £7 |
| Incremental cost- effectiveness ratio (ICER) (change in cost / change in effect) (£000's) | | (£10/1) = £10 | (£150/20) = £7.5 |

The three projects can be compared in many ways. First, in terms of their effects (however this might be defined), this may be 'lives saved', 'reduction in pain', 'blood pressure', 'satisfaction' or 'utility', etc. In this example project C has the largest effect (30). Second, they may be compared in terms of their costs. Depending on the costing perspective of this study projects may be compared in terms of cost to individuals, the health service, or society. In this example project A has the lowest cost (£50,000). Third, they may be compared in terms of a cost-benefit analysis. If the effect of these projects can be given (or translated into) a monetary value then the impact of these projects is calculated by a simple equation on a monetary scale, value of benefits minus the value of costs, this is labelled net economic impact or net benefit. In this example only project A (£54,000 - £50,000) has a positive net benefit (the others generate a loss for society). Fourth, they may be compared in terms of the average cost-effectiveness ratio. This is simply the average cost per unit of effect (cost divided by effect). Again the effect may be defined in many ways, it may even

be in terms of QALYs, in which case this could be labelled a cost-utility analysis. In this example project A has the lowest average cost per unit of effect.

These are all possible ways of presenting data on these three projects but remember that the aim is to aid decision-making. Looking at cost or effect in isolation is partial analysis; monetary valuation of effects may be undesirable or impossible; and average cost-effectiveness may be meaningless in a policy context since it compares each project with a 'do nothing' strategy and not with alternative projects. In this example we already have an existing strategy, project A is current practice. Decision-makers need to know what is the change in cost and effect in project B and C compared with project A. It is therefore very important that the incremental cost-effectiveness ratio (ICER) is presented. This is the additional cost of achieving an additional unit of benefit (change in cost divided by change in effect). In this example project C is £150,000 more expensive than project A but generates 20 more units of effect, the ICER is lower than for project B.

"In this world of rational disorder the only certainty is change"

Attributed to Jean Paul Sartre

This highlights the importance of the margin in economic analyses. The key word is change. Decision-makers are interested in the change in resources or health status that different activities can bring about. Conceptually this can be demonstrated as follows. If we accept the aim is to maximise benefit ('healthiness') subject to our limited resources then there will always be a case for reallocating resources in our health system until we reach the optimal point which is given as:

$$MB(a)/MC(a) = MB(b)/MC(b) = MB(c)/MC(c)$$

Let us assume that there are only three interventions in the world (a, b, c). MB is marginal benefit, MC is marginal cost, hence the ratio (MB/MC) is the additional benefit generated per additional unit of cost (per £). Now, if this ratio is identical for all interventions there is no case for further reallocation of resources since it will not add any more benefit to the system. Suppose that: -

$$MB(a)/MC(a) > MB(b)/MC(b)$$

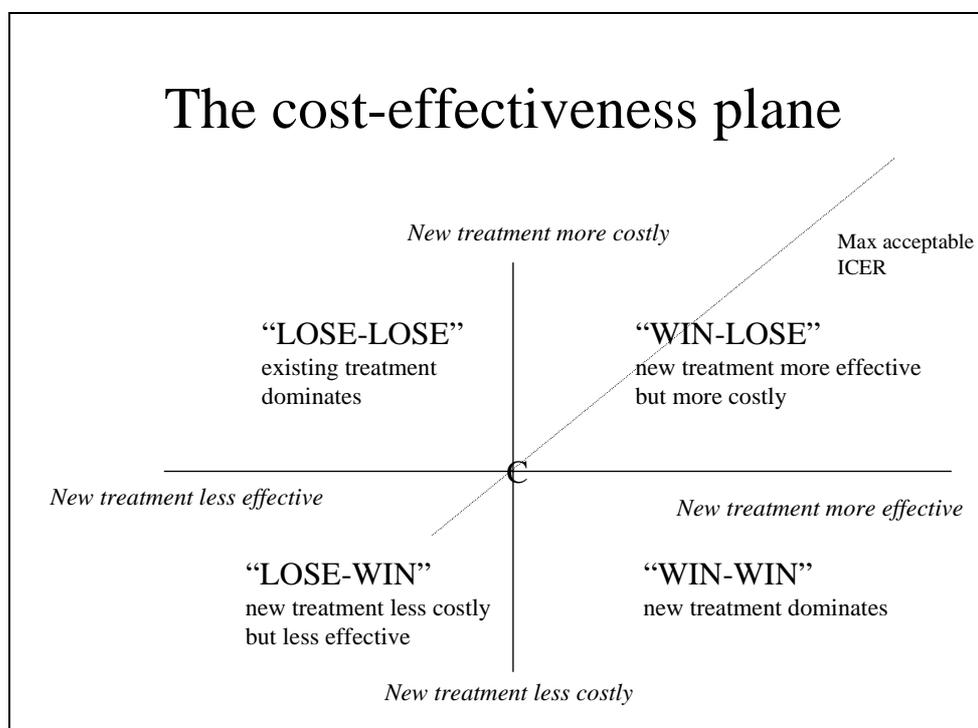
then there would be a case to reallocate since the benefit lost by withdrawing £1,000 from project B would be less than the benefit gained by reallocating that £1,000 to project A. The total benefit would therefore be increased.

Intuitively too it is clear that the margin is important. Life is about change. Very few decisions start with a 'green field site' or a 'clean sheet of paper'. Most inherit an existing allocation of resources. If health economic evaluation is to meet its objective of aiding decision-makers then it is essential that marginal costs and marginal benefits are considered in these analyses.

A useful way of presenting incremental cost-effectiveness analyses is by use of the cost-effectiveness plane (figure 5). On this graph the origin (0, 0) represents the existing intervention with which a new intervention is being compared. Using this

graph we can plot the change in costs and benefits brought about by the new intervention. The four quadrants have different policy implications. If the new intervention is located in the north-west quadrant (lose-lose) it has higher costs and lower benefits than the existing intervention and thus the existing intervention dominates. If the new intervention is located in the south-east quadrant (win-win) it has lower costs and higher benefits than the existing intervention and is thus the dominant strategy. The remaining two quadrants are a little more tricky. The decision-maker can have more benefit if they are prepared to pay more (north-east quadrant) and indeed they can save resources if they are prepared to give up some benefit (south-west quadrant). If these trade-offs are considered worthwhile the new intervention can still be labelled as cost-effective.

Figure 5 - The CE Plane

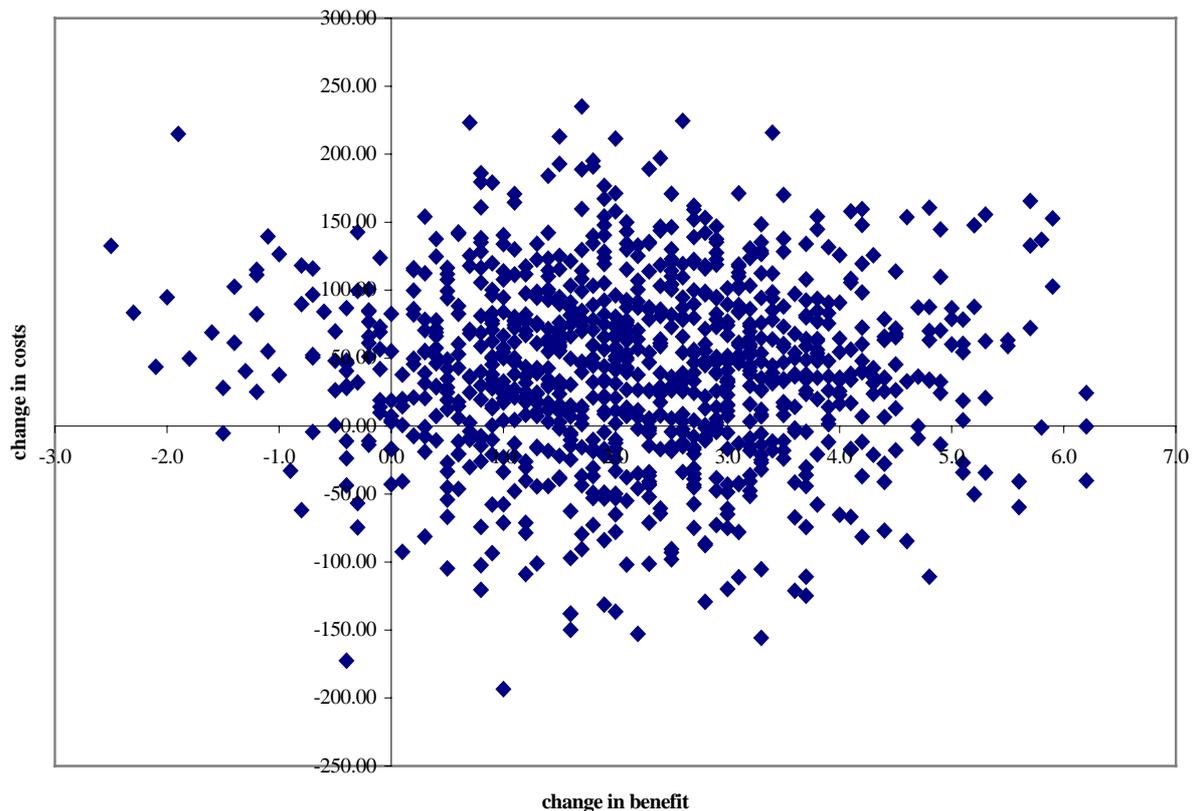


Dealing with Uncertainty

One further issue to consider in any evaluation is uncertainty (Briggs and Fenn 1998, Briggs and Gray 1999). Whilst all evaluations strive to be rigorous and systematic in their approach they will inevitably be subject to some uncertainty. The measurement of key cost and benefit variables is crucial to the result of an economic evaluation (i.e. this is the best use of resources). A change in the value of any of these variables might in fact reverse this result (i.e. one variable may be slightly lower than our observed measurement and in fact it is not the best use of resources hence our result would be wrong). Results are sensitive to the deterministic variables in the analysis. The crucial issue is just how sensitive? To test the level of uncertainty of our measured variables sensitivity analysis should be performed. We might, for example, want to vary the amount of nurse time we have estimated for a certain procedure, say,

double it from 1 hour to 2 hours. What effect does this have on our result, if any? It is wise to vary the most important and most uncertain variables within a plausible and justifiable range in order to test the robustness of the evaluation carried out.

Figure 6 - Bootstrapping cost and consequence data



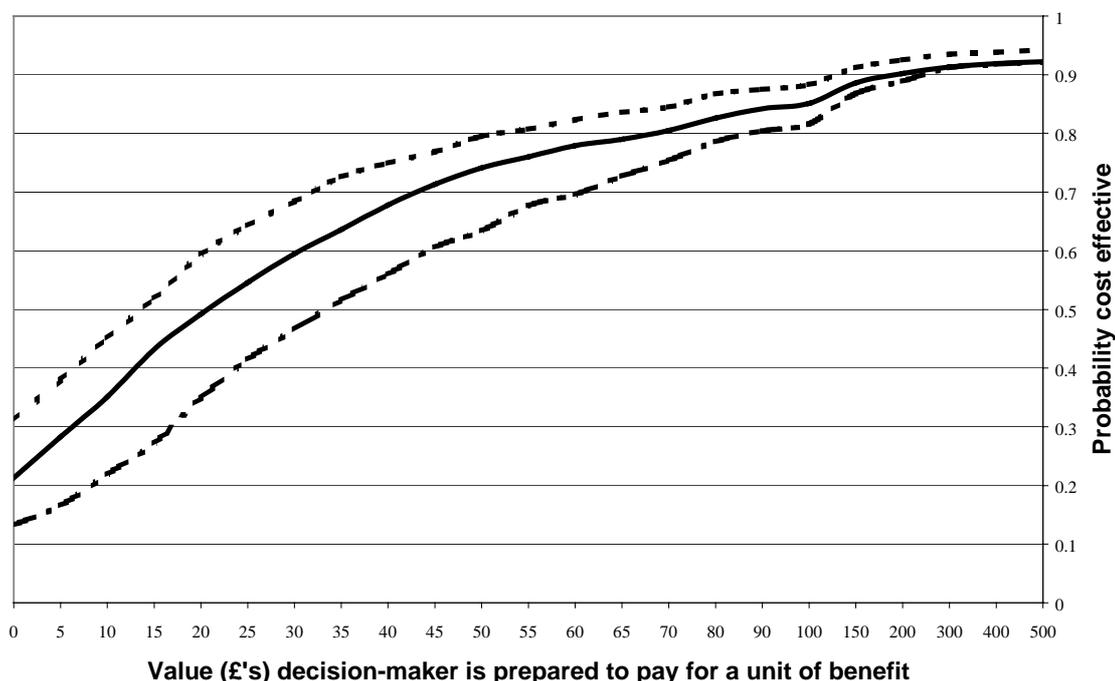
Sensitivity analysis can take many forms: one-way analysis, changing only one variable at a time; multi-way analysis, changing several variables; worst-case and best-case analysis; and threshold analysis, what values would variables have to reach to change the result?

Whilst cost and benefit estimates for an economic evaluation may be drawn from an observed distribution from which we can make statistical inferences and then carry out sensitivity analyses, the ratio statistic (the ICER) often receives none of this attention. Cost and benefit estimates are normally accompanied by confidence intervals reflecting the variability and the range of data. The ICER is given as a point estimate (Intervention group mean cost - control group mean cost) / (Intervention group mean effect - control group mean effect). Yet decision-makers need to know how likely it is that our estimated ICER reflects the 'truth'. Since the ICER is a ratio, producing confidence intervals is not straightforward.

A useful approach to resolving this problem of uncertainty around the ICER is to produce an acceptability curve (Van Hout 1994, Lothgren 2000) which decision-makers can then use. This can be generated by using a re-sampling technique called bootstrapping. This technique takes random samples of our cost and effect datasets and re-calculates the mean values. This is repeated many times (say 2000). Using the 2000 'bootstrapped' means for costs and effects an ICER can then be calculated for each. This provides us with a distribution of ICERs which can be plotted on a cost-effectiveness plane (figure 6).

The acceptability curve is constructed by saying, O.K. decision-makers can have extra units of benefit if they are prepared to pay more for it. So, how much are they prepared to pay? We do not know! What if they are prepared to pay a maximum of £10 for one unit of benefit? This point can be plotted on the CE-plane, if we then draw a line through the origin and this point, we have divided the graph into two planes. All points below this line (down to the right) can then be labelled as cost-effective given the decision-makers criteria. Since the 'bootstrapping' technique provided us with a distribution of ICER estimates, we can simply calculate the proportion of ICER estimates below and above the (cost-effectiveness) line. This will then tell us the likelihood (probability) that the intervention can be labelled as cost-effective when the decision-maker is prepared to trade-off this amount for extra benefit. So, in this example it might be that 90% of our distribution lays below our line of cost-effectiveness. This means that when the decision-maker is prepared to pay up to £10 for a unit of extra benefit there is a 90% chance that this new intervention can then be labelled as cost-effective compared to the existing intervention. The process is repeated using many different values that decision-makers will be prepared to pay for extra units of benefit. For each value the proportion of the distribution of ICER estimates under the line is calculated. The acceptability curve can then be drawn as in figure 7.

Figure 7 - Cost-Effectiveness Acceptability Curves



Sensitivity analyses can also be presented using acceptability curves. The dotted lines in figure 7 represent results when base-case assumptions are varied within a plausible range.

Concluding Comment

"...economics has made a positive contribution to health and medical care, and I believe that future contributions will be even greater" (Victor Fuchs, eminent Economist⁶)

Health economics is now a central tool for those who plan, provide, receive, or pay for health services. It is essential that those involved in this process are fully aware of and understand the concepts of health economic evaluation. Economic evaluations analyse the consequences of using health programmes, in terms of both their benefits and their costs, compared to competing alternatives. Various economic evaluation techniques exist to help provide decision-makers with information about value for money and ultimately to improve the impact of the resource we spend.

⁶ In: Folland et al (2001)

The following quick exercise may be useful to test your understanding of health economic evaluation.

Self-assessment: questions

True or False?

State whether the following are true or false.

1. It is possible for one intervention to be less clinically-effective than another but, at the same time, be more cost-effective.
2. In cases where detailed cost-effectiveness information is unavailable, clinicians should use the most effective option without question, irrespective of whatever its cost might be.
3. If the patient possess no capacity to benefit from a treatment, and the treatment would require the use of resources, it would be more cost-effective to do nothing rather than to treat
4. Clinical treatments – for example, cardiac surgery vs. drugs for asthma vs. psychiatric care – are simply different and not more or less important. Accordingly, treatments cannot be prioritised.
5. When combining quality and quantity outcome measures to calculate QALYs, it is conventional to attach more importance to quantity than to quality.
6. It is quite possible for an intervention to be both cost-effective and unaffordable at present.
7. Cost-benefit analysis converts all health consequences and benefits to a monetary value.
8. Cost-effectiveness analysis enables the decision-maker to make efficiency comparisons across very different clinical areas.
9. A QALY is a measure that includes the number of lives saved and quality of life.
10. In health care efficient use of resources means maximising the health benefits from a given input of health care spending.

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Websites:

- Health Economics links -

<http://www.york.ac.uk/res/herc>

- Health Economics Research Group, Brunel University -

<http://http1.brunel.ac.uk:8080/dept/herg/home.html>

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Glossary

| | |
|-----------------------------|---|
| Bootstrapping | A statistical approach for examining the uncertainty in cost-effectiveness analysis. It is a non-parametric simulation method used when the underlying data have a skewed distribution. |
| Cost-benefit analysis | Type of economic evaluation that measures costs and benefits in monetary units and computes a net pecuniary gain/loss. |
| Cost-effectiveness analysis | Type of economic evaluation that measures therapeutic effects in physical or natural units and computes a cost/effect ratio for comparison purposes. |
| Cost-utility analysis | Type of economic evaluation that measures therapeutic consequences in utility units (e.g. QALYs) rather than physical units. |
| Direct costs | Fixed and variable costs associated directly with a health care intervention. |
| Economic evaluation | A comparative analysis of two or more alternatives in terms of their costs and consequences. |
| Health economics | Application of theories, concepts and tools of economics to the topic of health and health care. |
| Incremental cost | The cost difference between health programmes under comparison |
| Indirect costs | Cost of reduced productivity resulting from illness or treatment. |
| Intangible costs | The cost of pain and suffering as a result of illness or treatment. |
| Marginal cost | The extra cost of one extra unit of product or service delivered. |

Appendix 1

Self-assessment: answers

Multiple choice questions

- 1) This is a straightforward question on the concept of opportunity cost and the problem of choosing between the alternative uses of scarce resources. A separate question of how best to produce this lunch would be an issue of technical efficiency. Which combination of ingredients in this lunch will maximise enjoyment (benefits), would be an issue of allocative efficiency. The additional cost of producing one more lunch would be the marginal cost. The answer is c).
- 2) Discounting may be used when the timing of costs and benefits of health programmes being compared differ. It is simply a way of adjusting for this differential timing. The main reason this is important is that people may have a differing rate of time preference i.e. prefer benefits now rather than in the future and costs in the future rather than now. Whilst the presence of inflation and bank interest rates may also make discounting seem logical they are not the principle reason to do it. This concept should not be confused with any economies of scale arguments or the removal of certain variables from an analysis. The answer is d).
- 3) Allocative efficiency is about finding the optimal mix of services that deliver the maximum possible benefits in total, across all health care activities, within the given budget. Technical efficiency is about the best way to achieve an outcome that we already perceive is desirable. We may need to be aware of the equity implications of decision based on allocative efficiency. The answer is b).

Self-assessment: answers

True or False?

True: 1,3,6,7,10

False: 2,4,5,8,9

Short answer questions

- a) The issue to be addressed here is one of technical efficiency, how we should run a screening programme (i.e. what strategy? Which women? How often? etc.) and not whether we should do it, since this has already been decided. The aim then would be to maximise health output for the given resource input of up to £500,000. The most informative type of economic evaluation would be cost-effectiveness analysis, 'cost-minimisation' analysis may even be sufficient. Given the stated purpose of the screening programme, useful measures of effectiveness for economic evaluation might be: the number of women screened, the number of cancers detected or perhaps the number of treatable cancers detected. Hence an appropriate cost-effective ratio with

which to compare strategies for breast cancer screening would be cost per treatable cancer detected. In order to present effectiveness in terms of lives saved or life years gained further information about the effectiveness and cost-effectiveness of available breast cancer treatment programmes would also be required.

- b) Again this is an issue of technical efficiency. Up to £800,000 will be spent on breast cancer screening, an evaluation will help decide how best to do this screening. Under this scenario we are not given any clear objectives for the screening programme. A cost-effectiveness analysis could be used to compare different strategies as a uni-dimensional outcome measure may be sufficient within this one clinical area. Due to the lack of stated objective we would not be able to decide the most appropriate effectiveness measure. More information is required.
- c) This is an issue of allocative efficiency since an evaluation is required to help decide whether to allocate resources to breast cancer screening or some other intervention. This will certainly involve comparing across clinical areas and possibly with other more diverse economic activities. A cost effectiveness analysis would be useful if a common outcome measure can be found (eg. lives saved). A cost utility analysis would provide better comparability across diverse health interventions but quality of life may not be relevant in the area of breast cancer screening. A cost benefit analysis might be the appropriate design. Benefits from a screening programme could be presented in monetary terms such as the savings from medical care avoided etc. Valuing the information and reassurance to the women in this framework will however be problematic.
- d) A cost effectiveness analysis would be sufficient to answer this question if we can agree on a single outcome measure. Under this scenario the objectives are not stated so more information would be needed. (i.e is it intended to screen as many women as possible, detect the most cancers, or what?) Cost-effectiveness ratios could be compared for different age groups to find the most efficient target group for the screening.
- e) The answer to this question is exactly the same as d). only timing replaces the age of the women.
- f) To compare across these disease groups the 'common currency' of CUA or CBA is required. Since the interventions listed have implications for quantity and quality of life CUA may be most appropriate. This is especially true given the inherent problems of measuring these types of benefits in monetary terms.
- g) Instead of maximising health we are now asked to maximise benefit. Since utility is a measure of benefit then a CUA is still appropriate.