Economic Evaluations (EE) alongside Randomised Controlled Trials (RCT).

1. Introduction

Only limited resources are available for tackling health care interventions. It is important that these resources are used to maximise benefits (i.e. used efficiently). Economic evaluation provides guidance on how best to use resources: it is a systematic analysis that make easier to identify the relevant alternatives, it makes explicit the viewpoint of the analysis, and it attempts to measure benefits and costs differences between relevant alternatives.

2. What is an economic evaluation?

Drummond et al. (1997) defines it as “the comparative analysis of alternatives courses of action in terms of both their costs and consequences”.\(^1\)

Johnston et al. (1999) stressed the key advantage of using clinical trials based economic evaluations as the fact that clinical trials provide the opportunity to collect and analyse patient-specific resource use and costs for a given patient population in the same conditions in a single study.\(^2\) This can be valid also for other outcomes such as effectiveness, benefits or utilities. This information may not be available at any other time.

The main interest for economic evaluation is the assessment of cost-effectiveness for an intervention in a given population. Because of this, the gold standard evaluation alongside RCT would be:\(^3\)

- Conducted in naturalistic settings
- With the interventions as close as it would be used in usual care
- With the sufficient power to assess those results in for a wide range of clinical settings and clinical indications within the intervention will be used
- With the adequate follow-up to assess full impact of the intervention
- With the proper time frame for decision-making and dissemination

These gold standard conditions may be thought of as those that define a pragmatic RCT.

There are several issues to be addressed when undertaking an economic evaluation in any area (health care or any other); some of them particular to economic evaluation alongside RCT. The general economic evaluation issues are briefly presented in Section 4, the different types of economic evaluation are presented in Section 5, the steps involved in conducting an economic evaluation in Section 6, while a list

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of specific aspects of economic evaluation alongside RCT are presented in Section 7. Section 8 presents
the 10 points checklist of Drummond for appraising an economic evaluation and Section 9 a list of
potential interesting references. Finally, a Glossary of term is included in Section 10.

3. Issues arising in any Economic Evaluation

Economic evaluation should always involve the quantitative comparison of two or more interventions in
terms of their cost (the resources that they use) and benefits. Some basic matters should be clearly defined
before starting any economic evaluation, namely, the objective of the economic evaluation, the
perspective from which the economic evaluation will be performed, and the type of economic evaluation
that will be developed.

3.1. Objective of the economic evaluation

The objective of the economic evaluation should be clearly stated. For instance, “to address the cost-
effectiveness of alternative A against alternative B”. In this way, the proper specification of the
alternatives cannot be avoided.

3.2. Perspective of the economic evaluation

The perspective of the economic evaluation is the point of view from which the evaluation will be done.
When defining the perspective of the analysis, the type of costs and benefits –direct and/or indirect– to be
collected is also defined. Perspective of the analysis can be:

- “Societal”, (direct costs and indirect costs will be collected)
- “Hospital” (all direct medical and non-medical cost are included)
- “Third party payer” (insurance companies’ costs are included)
- “The decision maker” (i.e. the local government; relevant cost for this will be collected)
- “Clinician” (those costs that are incurred by the clinician are included)
- “Patient” (costs incurred by patients –out of pocket payments that are not reimbursed– are
  included)
- Employer (cost relevant to the employer are collected)

4. Different types of Economic Evaluations:

The economic evaluation can be classified in terms of the way in which benefits are measured:

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http://www.york.ac.uk/inst/crd/report6.htm
• **Cost Minimisation Analysis**: when consequences between alternatives are known as equivalent, the analysis could be focused only on costs no matter which way consequences were measured.

• **Cost-effectiveness analysis (CEA)**: when the alternatives to be compared have a common type of health outcome it is possible to make the comparison in this specific unit of measure (i.e. life years saved, number of cases detected by a screening test, etc.).
  
  o **Cost-consequences analysis**: this is a particular type of CEA when there are a number of relevant measures of effectiveness and none of them is outstanding, it is possible to present cost and consequences in a disaggregated form. This avoids the issue of collapsing in a single unit of measure all the effectiveness measures and leaves to the decision maker the possibility to decide the weight to be given to each of them.

• **Cost-utility analysis**: some economists are not quite happy to measure consequences in monetary units as it is done in Cost Benefit Analysis (see below), and prefer to measure them in other general measure called “utility”. This measure tries to reflect quantity and quality of life changes as a result of the interventions. Is a general measure and, theoretically, makes it possible to compare alternatives of different programmes across different treatment areas (i.e. **Quality Adjusted Life Years** or **Health Years Equivalents** –see Glossary–).

• **Cost-benefit analysis**: when attempts are made to measure all costs and consequences in commensurate unit, usually monetary units (pounds, or any other currency). This type of economic evaluation can potentially compare interventions in any area as the unit of measurement is the same. One of the main methods used to assess benefits it the concept of **Willingness to Pay** (see Glossary).

5. Steps in an economic evaluation

The economic evaluation can be seen as a six steps process (Glick et al. 2001)

1. Quantification of outcomes (effectiveness, utilities, benefits, or any other measure of effectiveness)

2. Quantification of the costs (direct costs and also indirect costs if a societal perspective is adopted)

3. Assess whether and how much means costs and outcomes differ between alternatives

4. Comparison of the magnitudes of differences in incremental costs and incremental outcomes (report cost-effectiveness ratio or net-health benefits)

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5. Evaluation of the precision of these comparisons (reporting confidence intervals for the measures presented in step 4)

6. Addressing of other sources of uncertainty in a sensitivity analysis.

5.1. Costs

The definition of costs that economists have is not the same as that used by accountants. Accountants define direct costs as those that can be assigned to a particular office or job; all other costs that are “shared” by different departments will be defined as overhead costs (front door Security in hospital is shared by all the departments in that hospital and its costs could be split between them). Economists define all these costs as direct costs that result from the activity. Indirect costs is use to denote the time of patients and their families consumed or freed as a result of the intervention. That time has an opportunity cost (the best alternative use of that time) and usually it is associated with productivity losses (time expended in other activities that could be expended at work).

Johnston et al. (1999) define the different categories for cost as Health service costs, Non-health service costs and Non-resource costs. Within the first category they include the direct cost of the whole intervention, the costs of other illnesses that are not associated with the intervention, future health services costs and trial driven costs (not very important in pragmatic trials). In the second they include costs incurred by other public sector budgets, patient’s travel costs, other out of pocket expenses by the patient, informal care costs, patient’s time costs when receiving treatment, productivity costs associated with morbidity and mortality, and future non-health service costs. Finally, the most common type of non-resource costs is transfer payments (flows of money from one group of society to another that involve no resource consumption –e.g. social security payments–).

It should be noted here that, no matter the cost classification adopted, the criteria for including a particular cost/resource used into the costs of a particular arm is attribution. In other words, can this particular cost be attributed to this intervention? It may be easy to find examples of unattributable costs when looking for future non-health service cost category. The problem here is to know, in advance, which cost are or are not attributable to the intervention in order to collect them or not.

Examples of these costs are listed in sections 5.1.1 and 5.1.2

5.1.1. Direct costs

- Hospital Care (health service costs):
  - Hospital (hotel) care
  - Specialised care (e.g. intensive care)
  - Medical staff costs
  - Nursing staff costs

See Productivity costs in the Glossary
5.1.2. Indirect costs (non-health service costs)

- Patient and family
  - Travel costs (to hospital or clinic)
  - Informal home ‘nursing’
  - Out-of-pocket expenses (e.g. OTC medicines, special diet, mechanical aids, house modifications)

5.2. Benefits

There are many instruments to assess outcomes from a RCT that could be used in economic evaluations. Some of them refer to a specific aspect that could be modified in the participants with the intervention; others refer to survival time; finally, could be the case that the important change is the quality of life instead of or as well of survival time as a result of the intervention.
In economics evaluations the quality of life data is usually described as disease specific and general health profiles (Drummond (1994)). The former focuses on specific aspects of the disease or the intervention in the trial. It has the advantage of being able to show differences in outcomes and the disadvantage of not being easily comparable to results from other studies. The latter cover broader relevant domains of health related quality of life as physical functioning, ability of self-care, pain, psychological status and level of social integration (i.e. EuroQol EQ-5D, HUI). The main advantage of these is that they can be used to make comparisons between interventions in many different disease areas.

Economists are interested not only in the description of these health states but also in an individual’s preferences for one state instead of the other. A number of approaches have been developed to assess these preferences:

- **Rating scale measurements (or Visual Analogue Scales):** a line is presented to the person with clearly defined endpoints; the most preferred health state is placed on one end and the least preferred at the other. The other health states are placed between these two and the “distance” between the alternative health states corresponds to the difference in preference as perceived by the person.

- **Time trade-off measurements (TTO):** respondents are asked to consider a health state and to assess how many years on that state are they willing to give up in order to return to the full health state for the rest of their life. The worst the particular health state the more years the person is willing to trade for full health state life. The number of years traded gives a notion of the relative preference for the different health states. This approach was developed by Torrance et al. (1972) as an easier to understand method for subjects than standard gamble approach.

- **Standard gamble (SG):** subject is offered the certainty of living the rest of their life in a particular health state or a gamble (e.g. treatment, surgery, etc.), which could return him to full health state with probability p or to immediate death with probability (1-p). The probability p is varied until the individual is indifferent between the gamble and the certainty. The worst the health state the bigger the gamble the individual will be willing to accept. The “indifferent” probabilities will give a relative valuation of the particular health states. While TTO approach generates ‘values’ the SG generates utility weights. Finally, SG is based on the axioms of the expected utility theory and as a result is believed by many economists to be the gold standard as the visual analogue and the TTO have little or no basis on economic theory.

- **Contingent valuation (CV) or Willingness to Pay (WTP):** respondents are required to think about the contingency of an actual market for a particular program or health benefit and to reveal the maximum that they would be willing to pay for such program or benefit. This is usually referred to as a “stated

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9 http://www.euroqol.org/
10 http://www.fhs.mcmaster.ca/hug/
preference” method as participants are asked to address an actual value (rather than inferring a value from the answer they give – “revelled preference methods”)

- **Conjoint analysis or Discrete Choice Experiments (DCE):** this approach uses ranking, rating or pair wise comparisons exercises to estimate the relative weights that people attach to different attributes of a commodity. It can be used for the calculation of: the QALY gains from different interventions when the various dimensions of health are used as attributes; to calculate the implied willingness to pay for those interventions when money is included as attribute; and express the value of different attributes in terms of another.\[\text{15}\]

5.3. Estimation of Incremental Cost Effectiveness Ratio (ICER)

Once the analyst has costs and outcomes for each alternative (i.e. for each arm in the trial), the appropriate comparison between alternatives is to calculate the incremental costs and compare them to the incremental effectiveness outcomes. The usual from for this is to calculate the Incremental Cost Effectiveness Ratio (ICER). If A is the more cost effective treatment currently known and B is the new alternative treatment, then, \(C_A\) and \(C_B\) are the costs of alternative A and B, respectively; and \(E_A\) and \(E_B\) are the effectiveness of A and B respectively, then the ICER is given by:

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\text{ICER} = \frac{C_B - C_A}{E_B - E_A}
\]

This gives the notion of the cost that should be assumed for gaining one unit of output. In other words, if one of the alternatives is the usual practice, then it will tell us how much it will cost to gain a unit of outcome when moving from the usual practice to the alternative considered in the study.

The figure illustrates this, the relevant comparison is not the average cost per unit of effectiveness of the alternatives (namely, the slope of the line from the origin to each point either A or B), but to compare the incremental cost and the incremental effectiveness when moving from point A to B (The ICER of B compared to A is the slope of the line between point A and B).

5.4. Sensitivity Analysis

There are many sources of uncertainty around economic evaluations. Briggs and Gray (1999) state that “uncertainty in economic evaluation is pervasive, entering the evaluative process in every stage”. They relate this overall uncertainty to the uncertainty in four broad areas; 1) the appropriate analytic methods employed in conducting the evaluations (i.e. methods of measurement and valuation of costs and effectiveness), 2) the data requirements of the study (i.e. resource use, outcome consequences which vary between individuals; unit costs which might vary between centres and quality of life weights that can vary between populations), 3) the extrapolation of study results (i.e. from primary data to intermediate health outcomes to final health outcome used in economic evaluations); and 4) the generalisability of those results (i.e. whether results from one study could be generalised to other group of patients or other settings).

Sensitivity analysis (SA) involves “systematically examining the influence of the variables and assumptions employed in the evaluation” (Briggs and Gray (1999)) for the estimated results and is the way economists deal with uncertainty in economic evaluations. Four main types of SA can be distinguished: 1) Simple SA; 2) Threshold analysis; 3) Extreme scenario analysis; 4) Probabilistic SA.

A simple SA can be one-way or multiway. In the former, a particular component (source of uncertainty) in the model is chosen and its value changed, while the other components remain constant. In multiway SA more than one component are varied at the same time. The threshold analysis tries to identify a critical value for a parameter for which conclusions may change and it is useful when the parameter is indeterminate. However this form of SA can only be used for continuous variables. The extreme scenario analysis identifies the best and the worst possible values for the parameters and repeats the analysis with these sets of values; it is particularly useful when the analyst has an idea of the baseline value for one or a more variables and a possible range but has no idea of the distribution. Finally, in a Probabilistic SA a distribution is attached to the variables in the model and a Monte Carlo simulation run; each simulation picks up values from the range and distributions specified for all the variables. This simulation is run for many times and a distribution of the results is obtained. Finally, from this distribution the variance of the results can be estimated.

5.5. Discounting

There is consensus in economics that a unit of money (imagine £1) today is not the same as the same unit of money in the future. For instance, there might be certainty about having £1 in my pocket today and uncertainty about a “promise” of money in the future. But even if there is certainty that the unit of money will be in my pocket in the future there might be uncertainty about what I will be able to do with that money in the future (risk of death or any other event that could make unlikely that I could benefit from that money in the same way that I can do it today). For this and other arguments it is said that individuals have time preferences. They prefer money today than money in the future; they prefer some events to happen in the present and some other in the future.

Imagine a person who is indifferent between £10 today or £11 in a year time. In this example, his preference rate is 10% (£1/£10). Theoretically, when the preference rate is known, it is possible to “move” money from the present to the future and from the future to the present, with no damage or extra
benefit to the individual, as he will be indifferent between these two alternatives. The important point for economic evaluation is that money today and money tomorrow cannot be directly compared, as they do not mean the same for the individuals and/or society. Therefore, all costs and consequences, regardless of the units they are expressed in, should be reported for a common point in time before any comparison is made.

There is a huge debate on different issues about discounting in economic evaluation. For example: which discount rate should be used in economic evaluations?; should health outcomes be discounted?; should the same discount rate be used for costs and outcomes?17

The usual practice is to discount costs and outcomes. The Washington Panel recommends to use the same discount rate for costs and outcomes but to relax this assumption in the SA.18 They also said that because large quantities of CEA have used the traditional rate of 5% and 3% they recommend using these rates. On the other hand, the National Institute for Clinical Excellence in UK recommends, “Future outcomes and costs must be discounted to reflect social time preferences and social opportunity costs of resources.”19 The conventional view is that benefits and costs should be discounted at the same rate. The current recommendation of the Department of Health and the National Assembly of Wales is that costs should be discounted at 6% per annum and benefits at 1½%. To maintain consistency with appraisals undertaken elsewhere within the NHS these values should be used in the base case analysis of evaluations in submissions to the Institute. Sensitivity analyses should also be carried out using, amongst others, the combinations 6% costs and 6% outcomes, and 6% costs and 0% outcomes.20

6. Issues arising particularly in ECONOMIC EVALUATION alongside RCT (section based on Glick H.A. et al. (2001)21)

Glick at al. 2001 stress some issues to be considered when designing an economic evaluation alongside RCT. Those are:

1. Plan the evaluation – write the protocol. This should define hypothesis and objectives, endpoints, a description of how endpoints would be constructed (e.g. multiplying resources with unit costs to obtain total costs). In addition, pilot work should test a data collection instruments and procedures and seek to identify potential covariables of costs and effects.

2. The proportion of total costs that should be collected in the trial must be defined. If data on costs are going to be collected prospectively then the goals are to measure services that make up a

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large portion of the difference in treatment between the study subjects randomised to the different groups and to measure services that make up a large portion of the total cost of care.

3. The delivery setting in which the data will be collected must be defined. If large amount of care are given in settings other than that in which the intervention were given or there are reasons to believe that the utilisation of these settings could differ between groups, then the extension of data collection efforts to these setting should be considered. For instance, surgical treatment is given in hospital but follow-up care may be given by the family doctor.

4. Utilisation in other areas must be identified. In the example given above, the records of a family doctor might be a good source of data. In other cases the information will be collected from the study subjects (self-reporting) or the study subjects proxies (e.g. family or carers). How often a study subject or their proxies should be contacted, if they are given any memory aid (i.e. a diary), and who should collect these data (i.e. research staff or a contract research organisation) are issues that should also be defined.

5. Ideally, data should be collected from all subjects in the study; at a minimum, from a random sample of all study subjects.

6. The form in which the data will be collected must be defined. In some cases administrative data (e.g. hospital records) are available and then resource utilisation and costs can be easily collected. If this is not the case, then usually health professionals record the resources used by the study subjects and costs are calculated by multiplying these with the corresponding estimation of unit costs. These unit costs estimations are often collected outside the trial as people that collect clinical data frequently feels uncomfortable collecting cost data as it is not readily available. Additionally, discrepancies between costs and health care prices are not uncommon and may not be easy to decide if prices are good proxies for costs. For example, the price for volunteer carers might be zero but the opportunity cost for them might not be zero.

7. Which unit costs estimates should be used for the study? The first choice should be to multiply centre-specific unit costs estimates (e.g. costs relevant to the hospital) with centre-specific quantities. When these unit costs estimations are not available estimations for similar centres and countries should be used. Practically, many investigators used average of the available unit costs.

8. For time limited trials of lifetime therapies: how will long-term results be projected? For time limited trials it is unclear that differences in costs and effects as well as the magnitude of those differences will vary or continue the same in the future. Decision analytic models could be used to address this issue.

7. Appraising an economic evaluation

Drummond et al. (1997) provided a list of 10 points to consider when critically appraising an economic evaluation and when writing an economic evaluation it is worth considering how well the proposed design protocol will meet the critical appraisal criteria.

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22 See Decision Analysis in Glossary
The 10 points of Drummond et al. (1997) are:

7.1. Was a well-defined question posed in answerable form? Did the study examine both the costs and effects of the service(s) or programme(s)? Did the study involve a comparison of alternatives? Was a viewpoint for the analysis stated and was the study placed in any particular decision-making context?

7.2. Was a comprehensive description of the competing alternatives given (i.e., can you tell who did what to whom, where, and how often)? Were any important alternatives omitted? Was (Should) a do-nothing alternative (be) considered?

7.3. Was the effectiveness of the programme or service established? Was this done through a randomised, controlled clinical trial? If so, did the trial protocol reflect what would happen in regular practice? Was effectiveness established through an overview of clinical studies? Were observational data or assumptions used to establish effectiveness? If so, what are the potential biases in results?

7.4. Were all important and relevant costs and consequences for each alternative identified? Was the range wide enough for the research question at hand? Did the costs cover all relevant viewpoints? Were capital costs, as well as operating costs, included?

7.5. Were costs and consequences measured accurately in appropriate physical units? Were any of the identified items omitted from measurement? If so, does this mean that they carry no weight in the subsequent analysis? Were there any special circumstances (e.g., joint use of resources) that made measurement difficult? Were these circumstances handled appropriately?

7.6. Were costs and consequences valued credibly? Were the sources of all values clearly identified? Were market values employed for changes involving resources gained or depleted? Where market values were absent (e.g., volunteer labour), or market values do not reflect actual values (such as clinic space donated at a reduced rate), were adjustments made to approximate market values? Was the valuation of consequences appropriate for the question posed (i.e. has the appropriate type or types of analysis - cost-effectiveness, cost-benefit, cost-utility - been selected)?

7.7. Were costs and consequences adjusted for differential timing? Were costs and consequences that occur in the future 'discounted' to their present values? Was any justification given for the discount rate used?

7.8. Was an incremental analysis of costs and consequences of alternatives performed? Were the additional (incremental) costs generated by one alternative over another compared to the additional effects, benefits or utilities generated?

7.9. Was a sensitivity analysis performed? If data on costs and consequences were stochastic, were appropriate statistical analyses performed? If a sensitivity analysis was employed, was a justification provided for the ranges of values (for key study parameters)? Were the study results sensitive to changes in the values (within the assumed range for sensitivity analysis, or within the confidence interval around the ratio of costs to consequences)?
7.10. Did the presentation and discussion of study results include all issues of concern to users? Were the conclusions of the analysis based on some overall index or ratio of costs to consequences (e.g., incremental cost-effectiveness ratio)? If so, was the index interpreted intelligently or in a mechanistic fashion? Were the results compared with those of others who have investigated the same question? If so, were allowances made for potential differences in study methodology? Did the study discuss the generalisability of the results to other settings and patient/client groups? Did the study allude to, or take account of, other important factors in the choice or decision under consideration (e.g., distribution of costs and consequences, or relevant ethical issues)? Did the study discuss issues of implementation, such as the feasibility of adopting the ‘preferred’ programme given existing financial or other constraints, and whether any freed resources could be redeployed to other worthwhile programmes?
8. References


http://www.euroqol.org/

http://www.fhs.mcmaster.ca/hug/


8.1. Some other potentially interesting references:

8.1.1. Journal articles


Donaldson C. Hundley V. McIntosh E. Using economics alongside clinical trials: why we cannot choose the evaluation technique in advance. Health Economics. 5(3):267-9, 1996 May-Jun


8.1.2. Some Economic Evaluation issues in BMJ:
Economics notes: Economic evaluation: an introduction
James Raftery

Economics notes: Perspectives in economic evaluation
Sarah Byford and James Raftery

Economics Notes: Types of economic evaluation
Stephen Palmer, Sarah Byford, and James Raftery

Economics notes: Opportunity cost
Stephen Palmer and James Raftery

Economic Note: Cost of illness studies
Sarah Byford, David J Torgerson, and James Raftery

Economics notes: Costing in economic evaluation
James Raftery

Economics Notes: Measuring outcomes in economic evaluations
David Torgerson and James Raftery

Economics notes: Discounting
David J Torgerson and James Raftery

Guidelines for authors and peer reviewers of economic submissions to the BMJ
M F Drummond and T O Jefferson

New BMJ policy on economic evaluations
Dawn Craig, John Nixon, Nigel Armstrong, Julie Glanville, Jos Kleijnen, Michael Drummond, Tracy Roberts, Alastair M Gray, Andrew Briggs, Philip Clarke, Charles Normand, Christopher J McCabe, Jennifer Roberts, and Richard Smith

Effectiveness, efficiency, and NICE
Mark Sculpher, Michael Drummond, and Bernie O'Brien

Evaluating the BMJ Guidelines for Economic Submissions
Prospective Audit of Economic Submissions to BMJ and The Lancet
Tom Jefferson, MD; Richard Smith, MD; Yunni Yee, Msc; Mike Drummond, DPhil; Mark Pratt; Rajendra Gale, MD JAMA 1998;280:275-277. [http://jama.ama-assn.org/cgi/reprint/280/3/275.pdf]
8.1.3. Other Website based resources

http://www.cche.net/usersguides/economic.asp

http://w3.whosea.org/healthreport/pdf/paper04.pdf


Etext on Health Technology Assessment (HTA) Information Resources (NLM/NHCHSR)


Henry Glick, University of Pennsylvania. Economic Evaluations in RCT. April 10, 2001
http://www.uphs.upenn.edu/dgimhsr/CTECN401.PDF

http://www.icmpe.org/test1/journal/issues/v1i1/v1i1text03.pdf

The CEA Registry: standardizing the methods and practices of cost-effectiveness analyses (formerly the Harvard CUA Database). (http://www.hsph.harvard.edu/cearegistry/)

WHO-CHOICE (Choosing Interventions that are cost-effective) WHO Global Programme On Evidence for Health Policy: Improving Health System Performance
http://www3.who.int/whosis/menu.cfm?path=whosis,evidence,cea&language=english

WHO Economic Evaluations Workbook
http://www.who.int/substance_abuse/PDFfiles/economicevaluation.pdf

Pharmacoeconomics:

Economic Evaluation Database, UK:
Infopoints: NHS Economic Evaluation Database for healthcare decision makers
John Nixon, Boyka Stoykova, Jimmy Christie, Julie Glanville, Jos Kleijnen, and Mike Drummond
This glossary provides a short list of definitions of key words, particularly those used in the early stages of the course. The words below are used and explained elsewhere in the course, but this list is offered for those who would find it useful to have all the words defined in one place. The following definitions and discussion are short and this glossary is no substitute for the fuller discussion of these terms which appears in the course materials. (Terms highlighted in bold in the definitions in the glossary are defined elsewhere is the glossary.)

Further terms and definitions can be found in Kielhorn A. and Graf von der Schulenburg J.-M. The health economics handbook, Adis International, Chester.

**Allocative efficiency** - Assesses competing programmes with competing objectives and judges the extent to which they meet objectives. An allocation of resources such that no change in spending priorities could improve the welfare of one person without reducing the welfare of another.

**Bootstrapping** – When data on cost, utilities, etc is obtained from a number of sources the estimates are likely to vary. In order to provide the best estimate (and the uncertainty surrounding that estimate i.e. a confidence interval) of the true value statistical techniques are used. Because of the nature of the data used in economic evaluations standard statistical tests are rarely appropriate because of the assumptions they make about the distribution of data. The statistical technique of bootstrapping does not make these assumptions. Bootstrapping is sometimes described as being a type of probabilistic sensitivity analysis.

**Business Care Analysis** - The systematic examination of the relative advantages and disadvantages of alternative options in meeting specific health objectives before resources are committed to one or more programmes. Technique usually used in appraisal of capital developments in the NHS (previously referred to as option appraisal).

**Ceteris Paribus** - all things being equal.

**Condition specific QALYs** – In condition specific QALYs the health state description focuses on the characteristics of the condition being evaluated. It is therefore important that condition specific QALYs are not used in QALY league tables.

**Conjoint analysis** - see Discrete Choice Experiments

**Complements** – if the quantity demanded of good A increases as a result of an increase in the quantity demanded of another good B then the goods are said to be complements; i.e. they tend to be consumed together. An example might be doctor visits and prescription drugs.

**Consumer Surplus** - the extra value which consumers gain when the price they have to pay for a good is less than their willingness to pay. It is measured by the area under the demand curve above the price level.

**Contingent valuation** - see Willingness to Pay

**Cost** - The economic definition of cost (also known as opportunity cost) is the value of opportunity forgone, strictly the best opportunity forgone, as a result of engaging resources in an activity. Note that there can be a cost without the exchange of money. Also the economists’ notion of cost extends beyond the cost falling on the health service alone and includes costs falling on other services and patients.

In considering the production process, costs may be differentiated as follows:

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24 Any information required on this see [http://www.abdn.ac.uk/heru/teaching.hti](http://www.abdn.ac.uk/heru/teaching.hti) or send an email to heru@abdn.ac.uk
**Average costs** - equivalent to the average cost per unit i.e. the total costs divided by the total number of units of production

**Fixed costs** - those costs which, within a short time-span, do not vary with the quantity of production e.g. heating and lighting

**Incremental cost** - the extra costs associated with an expansion in activity of a given service.

**Marginal cost** - the cost of producing one extra unit of a service.

**Total costs** - all costs incurred in the production of a set quantity of service.

**Variable costs** - those costs which vary with the level of production and are proportional to quantities produced.

In considering health problems, costs may be differentiated as follows:

**Avoided costs** - costs caused by a health problem or illness which are avoided by a health care intervention.

**Direct costs** - those costs borne by the healthcare system, community and patients' families in addressing the illness.

**Indirect costs** - mainly productivity losses to society caused by the health problem or disease.

**Cost-benefit analysis (CBA)** - an economic evaluation in which all costs and benefits of a programme are expressed in the same units, usually money.

**Cost-consequence analysis** - A form of economic evaluation where costs are set against an array of different measures of effectiveness (e.g. fractures avoided, mortality, etc) which are not aggregated together. An overall valuation of the benefits is not attempted and their valuation is left to the decision maker. It is different from **Cost-effectiveness analysis** which concentrates on a single outcome measure (e.g. life years saved, pain free days).

**Cost-effectiveness** - the point at which the minimum amount of input (and therefore cost) is used to achieve a given output.

**Cost effectiveness acceptability curve** - a graphical representation of the percentage of replications within a simulation exercise where the incremental cost-effectiveness ratio is below a specific threshold for a cost per QALY e.g. 90% chance that the incremental cost per QALY is below £20,000.

**Cost-effectiveness analysis (CEA)** - an economic evaluation in which the costs and consequences of alternative interventions are expressed as cost per unit of outcome. CEA is used to determine technical efficiency i.e. comparison of costs and consequences of competing interventions for a given patient group within a given budget. See also Technical Efficiency, Incremental cost-effectiveness

**Cost-minimisation analysis (CMA)** - an economic evaluation in which consequences of competing interventions are the same and in which only inputs i.e. costs are taken into consideration. The aim is to decide the least costly way of achieving the same outcome.

**Cost-utility analysis (CUA)** - a form of economic study design in which interventions which produce different consequences, in terms of both quantity and quality of life, are expressed as 'utilities'. The best known utility measure is the 'quality adjusted life year' or QALY. In this case, competing interventions are compared in terms of cost per utility (cost per QALY).
Cost of illness study - a study which itemises, values and sums the costs of a particular problem with the aim of giving an idea of its economic burden. A cost of illness study is not an economic evaluation.

Decision analysis - Decision analysis splits the investigation into components small enough to be readily understood and analysed. These components are then used to model the problem's essential elements. Under this methodology, the possible chains of events (from initial choice of intervention through chance events and final outcomes) are identified in a tree structure that clearly specifies their sequence. Data are analysed by giving each possible event a valuation (either in terms of resource use, health outcome or both), and by weighting valuations for uncertain events by the probability of occurrence.

Demand - reflects the willingness to pay by an individual or individuals for a good or service. Other things being equal, the more an individual values a further unit of a good or service, the more he or she will be prepared to give up for it. An individual will continue to demand more of a good or service until the value he or she places on the marginal unit is equal to what s/he has to pay for it.

Demand Curve - a graphical depiction of the relationship between quantity demanded and the price of a good or service. Also referred to as the demand schedule.

Dependent Variable – a variable whose value is determined by reference to the value of one or more independent values. E.g. quantity demanded is a dependent variable whose value depends on independent variables such as price and income.

Diminishing Marginal Utility – describes the reduction in value (utility) to the consumer of additional quantities of a good or service compared with earlier quantities consumed.

Disability adjusted life year (DALYs) - a measure of 'burden of illness' developed by the World Bank in the early 1990s as a tool to measure the impact of disease. Using this measure life expectancy is adjusted for loss of healthy life which occurs as a result disability and premature death.

Discounting - A technique which allows the calculation of present values of costs and benefits which accrue in the future. Discounting is based on a time preference which assumes that individuals prefer to forego a part of the benefits if they accrue it now, rather than fully in the uncertain future. By the same reasoning, individuals prefer to delay costs rather than incur them in the present. The strength of this preference is expressed by the discount rate which is inserted in economic evaluations.

Discrete choice experiments - Discrete choice experiments are an attribute based method of benefit assessment that can be used to value health outcomes, non-health outcomes and process attributes. The technique estimates the relative importance of different attributes, the rate at which individuals trade between these attributes, and willingness to pay if a price proxy is included as one of the attributes. Discrete choice experiments have also been referred to in the literature as conjoint analysis

Economic Appraisal - see Economic Evaluation

Economic Evaluation - the systematic appraisal of costs and benefits of projects, normally undertaken to determine the relative economic efficiency of programmes. See cost-benefit analysis, cost-effectiveness analysis, cost-minimisation analysis, cost-utility analysis, cost consequence analysis.

Economies of Scale - the average cost of production decreases as the quantity of output increases.

Effectiveness - The contribution which a programme makes to individuals' utility or welfare.

Efficiency - Making the best use of available resources i.e. getting good value for resources. See also Allocative efficiency and Technical efficiency.
**Elasticity of Demand** – the percentage change in the quantity demanded divided by the percentage change in factors causing the change in quantity demanded such as price or income.

**Elasticity of Supply** - the percentage change in the quantity supplied divided by the percentage change in factors causing the change in quantity supplied such as price.

**Equilibrium** – the point at which quantity demanded and quantity supplied are the same and there is no impetus for these to change.

**Equity** - The degree to which some distribution or other is judged to be 'fair'. 'Fairness' involves a value judgement so e.g. 'greater equality' need not imply 'greater equity'.

**Ex ante** - Before.

**Ex post** - After.

**Externalities** - negative or positive utilities accruing to an individual from another person's consumption or production. For example, if the majority of a community is vaccinated against an infectious disease, the resulting herd immunity benefits those who have not been vaccinated.

**Friction cost method** - developed to overcome problems associated with the human capital approach to estimate productivity costs. Using this method, production costs are estimated on the basis of the time taken to restore production to the original level.

Generic QALYs – derived using general health state descriptions, such as the EuroQol classification system, and are applicable over a large range of interventions and conditions.

**Healthy years equivalents (HYEs)** – suggested as an alternative to QALYs. The advantage of HYEs is that they fully represent individual preferences without imposing restrictive assumptions associated with QALYs. HYEs are measured using a two-stage gamble technique where the health state is described to the respondent, along with the duration of the state, and the respondent is asked how many years of life in full health would be equivalent to this scenario. Given the criticism in the literature concerning the merits of HYEs from a theoretical perspective there are few CUA s which employ HYEs to evaluate the benefits within the economic evaluation.

**Human capital approach** – used to estimate productivity costs and assumes that if all workers of a given type are paid the same, then the average wage equals the marginal value product and therefore that the wage rate is a satisfactory measure of the value of production. This approach has been criticised as overestimating the true cost to society as productivity lost at the margin is likely to be lower than the average wage as it is usually the less important tasks which are forgone as a result of an absence. See also friction cost method

Incremental Analysis – The results of a Cost-effectiveness Analysis can sometimes be used to provide information about Allocative Efficiency. This can be performed by calculating the Incremental cost effectiveness ratio which is the extra cost of obtaining an extra unit of outcome in comparing one intervention to another. A value judgement is required to determine whether the Opportunity costs of the extra unit of outcome is worthwhile.

**Income Effect** – the increase (decrease) in the quantity demanded brought about by an increase (decrease) in real income when the price of a good decreases (increases).

**Independent Variable** – a variable whose values are predetermined and affect the value of a dependent variable.
**Inferior Good** – a good for which demand decreases as income increases; i.e. the demand curve shifts to the left.

**Margin** - The last unit of production or consumption - although often relates to change of more than one unit.

**Marginal Analysis (MA)** - The evaluation of the change in costs and benefits produced by a change in production or consumption of one unit i.e. examines the effect of small changes in the existing pattern of health care expenditure in a given setting.

**Marginal Benefit** - The value of benefit derived when output is increased by one unit.

**Marginal cost** - The extra cost (i.e. the change in total costs of production) which results when output is increased by one unit.

  - **Short-run marginal costs** are those costs related to the marginal unit when not all the inputs to the production process can be varied.

  - **Long-run marginal costs** are those costs related to the marginal unit when all or most inputs to the production process can be varied.

Thus, if a "short-run" problem is being considered (e.g. whether to carry out more varicose vein operations next month), then only some of the cost items will be variable (e.g. sutures) and many will be fixed (e.g. employment of consultant surgeons).

On the other hand if we are planning for an increase in the provision of in-patient care for the elderly over the next 10 years, then long-run marginal costs are appropriate and all inputs would be relevant, including, for example, the capital costs of geriatric beds.

**Marginal Cost Pricing** – a profit maximising rule for producers.

**Marginal Utility** – the additional utility obtained from an additional unit of a good or service.

**Markov model** - A Markov model is composed of a set of defined states of health between which a patient can move over successive time periods. Such a model is run using a hypothetical cohort of patients. Transition probabilities are used to allow a patient to move within and between these states of health. A patient can be in only one state of health at any time and can make only one transition per cycle. The cycle is a discrete time period spent in each state of health before transition to a successive state of health. A relevant time period is chosen for the length of the cycle and the cycles then link together to create a ‘Markov chain.’

**Meta-analysis** – A statistical method for numerically combining the results of different studies that report on the same outcome to derive an overall estimate of effect. The technique is frequently used to provide more precise and generalisable data on clinical effects than would be available from a single study.

**Modelling** - Modelling may take many different forms. It may refer to analytical techniques to synthesis cost and benefits of alternative interventions (see Decision analysis and Markov model). Alternatively, it may refer to attempts to derive information on aspects of an economic evaluation (see Meta-analysis).

**Monte carlo analysis** – A simulation method used in Decision Analysis and Markov Modelling. It is a way of dealing with uncertainty surrounding the precise magnitude of utilities, cost or clinical effects. With this approach each utility, cost, or probability of an event is assigned a probability distribution which describes the likelihood that the variable of interest has a particular value. The economic evaluation is run many times (typically 1000 plus) with the values of the utilities, costs and even probabilities being randomly generated from the probability distribution.
Need - has been defined in a number of ways but all definitions recognise that need is determined by some third party rather than by the person who is in need. It therefore reflects the values of, for example, the doctor, rather than the patient. ('Want' and 'demand' are, on the other hand, based on the individual consumer's preferences.)

Opportunity Cost - The notion of cost used in economics.

Option Appraisal – see Business Case Analysis

Perfect Competition – a market structure with a set of conditions that ensure that welfare is maximised. These conditions, which include perfect information and certainty, are rarely fully satisfied, particularly in the health care sector.

Production - the use of resources to produce goods and services.

Production Cost – the cost of the resources used to produce goods and services.

Profit – the difference between the production cost and the revenue received from selling goods and services.

Quality Adjusted Life Years (QALYs) - Quality Adjusted Life Years (QALYs) were developed to take account of the fact that an individual may be concerned with the quality of their life as well as the quantity of life. To estimate QALYs, expected life years gained from given health care interventions are estimated (usually by health care professionals) and combined with information on the quality of these life years (via the estimation of utilities or quality weights). For example, if a health care intervention results in a health state with a utility score (quality weight) of 0.85, and the individual would be in this health state for the remainder of life, say 10 years, then the number of (undiscounted) QALYs would be 8.5. If the number of QALYs without the intervention were 4, then the QALYs gained from the intervention would be 4.5 (8.5-4). The QALYs gained from one health care intervention may be compared with QALYs obtained from alternative health care interventions, as well as from doing nothing. Standard gamble and time trade off are used to estimate utilities (quality weights) within the QALY framework.

Randomised Control Trial – A type of primary study in which the interventions under investigation are randomly assigned to trial participants

Sensitivity Analysis - A technique which repeats the comparison between costs and benefits, varying the assumptions underlying the estimates. In so doing, sensitivity analysis tests the robustness of the conclusions by varying the items around which there is uncertainty.

Standard Gamble (SG) - a technique used to estimate utilities (quality weights) within the QALY framework. Utilities are estimated by presenting an individual with a choice between a certain outcome (B) or a gamble which may result in either a better outcome (A) than the certain outcome (with a probability p) or a worse outcome (C) than the certain outcome (with a probability 1-p). The certain outcome is always an intermediate outcome in the sense that the better outcome is always preferred to it and the worse outcome is less preferred to it. The probability of the best outcome is varied until the individual is indifferent between the certain intermediate outcome and the gamble. This probability is the utility or quality weight for the certain outcome. It is clear that if the certain outcome (B) is undesirable then the individual will be willing to take a treatment gamble even if the probability of the preferred outcome is very low. Thus, the quality weight for outcome (B) would also be very low. Hence, using the standard gamble technique, the more undesirable an outcome, the lower the probability is that the individual will accept the gamble, and the lower the utility score for the undesirable outcome.
**Substitute** - if the quantity demanded of good A increases as a result of a decrease in the quantity demanded of another good B then the goods are said to be substitutes; i.e. they tend to be consumed as alternatives. An example might be doctor visits and over-the-counter drugs from a pharmacist.

**Substitution Effect** – the increase (decrease) in quantity demanded brought about by a relative decrease (increase) in the price of a product.

**Systematic Review** – the use of formal and reproducible methods to identify, and qualitative or quantitative (see meta-analysis) synthesis of, all existing data on a specific topic.

**Supply** – the willingness of a producer to supply a product at a given price.

**Supply Curve** – a graphical depiction of the relationship between quantity supplied and price.

**Technical Efficiency** - Assesses whether a given output can be achieved by using less of one input while holding all other inputs constant. This concept is related to cost-effectiveness. *See also cost-effectiveness* and *cost-effectiveness analysis*.

**Time Trade-Off (TTO)** - The time trade-off technique has been developed as an alternative, and easier, method than standard gamble for estimating quality weights within the QALY framework. The approach involves presenting individuals with a choice between living for a period \( t \) in a specified but less than perfect state (outcome B) versus having a healthier life (outcome A) for a time period \( h \) where \( h < t \). Time \( h \) is varied until the respondent is indifferent between the alternatives. The quality weight given to the less than perfect state is then \( h/t \). Using the time-trade-off technique, the more undesirable outcome B (the outcome being assessed) is, the more years of life an individual would be willing to give up to be in the best outcome (A), and the lower the ratio \( h/t \) and in turn the quality weight of outcome B.

**Utility** - A term used by economists to signify the satisfaction or benefit accruing to a person from the consumption of a good or service. Health economists have used Quality Adjusted Life Years (QALYs), Willingness To Pay (also referred to as contingent valuation) and Discrete Choice Experiments (also referred to as conjoint analysis) to measure utility. As with Discrete Choice Experiments, WTP can be used to value health outcomes, non-health outcomes and process attributes.

**Willingness-to-pay (WTP)** - represents the monetary value an individual places on a good or service. The technique of WTP is based on the premise that the maximum amount of money an individual is willing to pay (sacrifice) for a commodity is an indicator of the utility or satisfaction to them of that commodity. In the absence of a market, hypothetical questions are asked to elicit WTP. Such hypothetical questions are referred to as Contingent Valuation Surveys. As with Discrete Choice Experiments, WTP can be used to value health outcomes, non-health outcomes and process attributes.