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The Economic Framework for Health Service Evaluation and the Role for Discretion

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The Economic Framework for Health Service Evaluation and the Role for Discretion

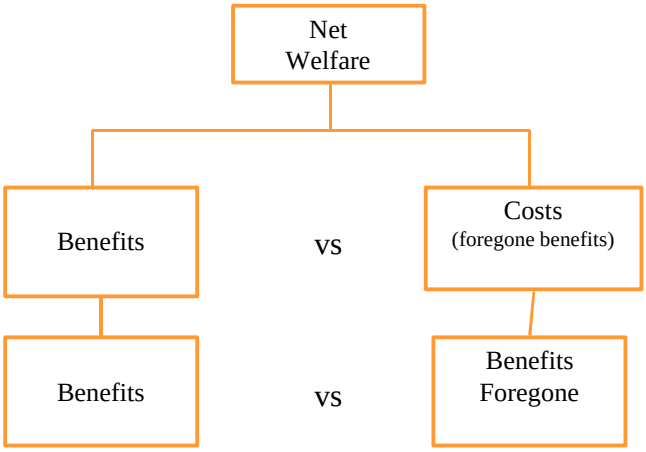
Introduction

The purpose of this paper is to introduce the economic framework for non-economists and to review various reasons why a government agency such as the Pharmaceutical Benefits Advisory Committee (PBAC) might deviate from the policy implications drawn from economic analyses as they are currently conducted. More specifically, it reviews some of the reasons why policy advice should not be based entirely upon the results incorporated in a league table constructed entirely from the costs, outcomes and the quality of life measurements that are usually included in an economic evaluation. The list of topics covered is necessarily incomplete and, in particular, there is no discussion of the tangled issues surrounding discounting of future health states. Rather, there is an emphasis upon issues which have received little discussion in the literature and have only recently been suggested for inclusion in economic evaluations. The discussion draws heavily upon work reported by Menzel et al (1999); Nord et al (1998); Ubel et al (1999); Olsen and Richardson (1998, 1999).

Economic Framework

In principle the objective of economics is to maximise social wellbeing or welfare when the resources that are available are limited. The distinctively economic contribution to this task arises from a recognition that, when resources are scarce, their use to achieve one objective necessarily prevents their use for some other purpose. This gives rise to the most fundamental and important concept in economics; *vis* the concept of an opportunity cost which is defined as the value of the benefits that are foregone (opportunities lost elsewhere) because of the use of resources to achieve a particular objective. This is shown in Figure 1. From this it is clear that at the broad conceptual level ‘cost benefit analysis’ – the comparison of costs and benefits could more accurately be described as ‘benefit-benefit analysis’.

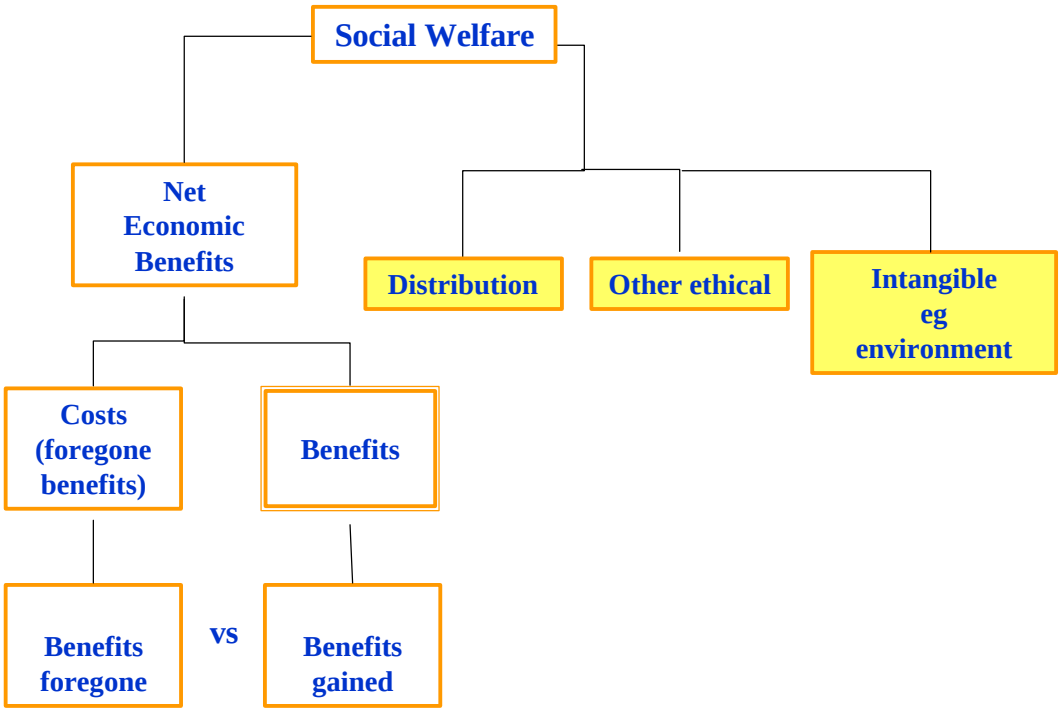
Figure 1 Simplified Economic Framework



At the level of abstraction embodied in Figure 1 the conclusion that costs and benefits should be compared for maximum wellbeing is a necessary truth as anything which adds or subtracts from wellbeing is included in the framework. Controversy only arises when these broad concepts are operationalised. In the practice of economic evaluation, the first step is to distinguish ethical distributional and other intangible benefits from those which are more readily measured; *vis*, those that are associated with the use of real resources. In Figure 2 where this distinction is illustrated, narrowly defined economic evaluation is often associated with the left hand side of the figure which deals with 'economic costs and benefits'.

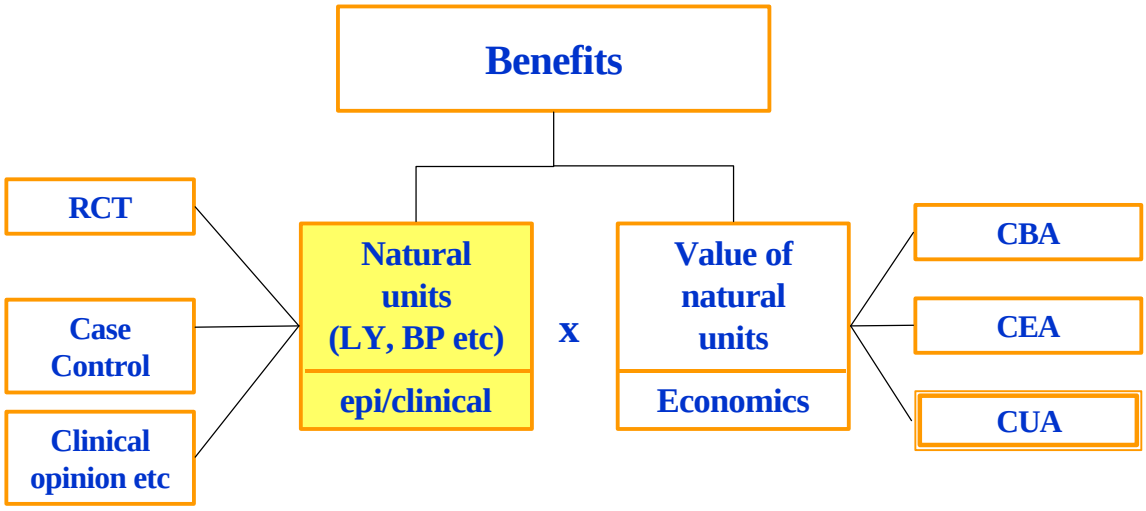
In principle, the distinction shown in this figure is artificial. Anything contributing to social welfare could be included in an analysis. In practice, this broad objective is difficult to achieve, firstly, because ethical and intangible considerations are often difficult to quantify but more fundamentally because there is often disagreement about which ethical values should be incorporated in an analysis and, in particular, it is unclear how benefits to different individuals should be combined to determine 'social welfare'. For this reason, good economic evaluation will simply note effects that are relevant to issues of social justice, equity and distribution. This does not always occur in practice. Many economic analyses are concerned with costs and benefits where there are no particular ethical issues – there is 'distributive' neutrality. In the domain of health – where compensation for loss is difficult, impractical or impossible – these issues, however, become of particular significance.

Figure 2 Structure of Economic Evaluation



A further important characteristic of economic evaluation is that economists are concerned with the measurement of value and have no particular expertise in the subject matter of the evaluation which, in the present context, is the effectiveness of various health related interventions. As a consequence, and as shown in Figure 3, an economic evaluation of a health program must be carried out in conjunction with, or based upon, a clinical or epidemiological analysis of the impact upon health as measured in natural units. As shown, the economic assessment is an addition to, and not a substitute for, this type of investigation and the reliability and validity of the final conclusion of an economic evaluation depends, in large part, upon the reliability and validity of the clinical trial upon which it is based.

Figure 3 Interface Between Economic and Epidemiological Evaluation



Three types of economic evaluation are shown in Figure 3. These are:

Cost Benefit Analysis: The defining characteristic of CBA is that benefits are measured in dollars. This permits a direct comparison of costs and benefits and, in the absence of any other relevant consideration, a decision regarding the desirability of the program. Various techniques have been developed for measuring the monetary value of health outcomes, the most commonly advocated being the willingness-to-pay for the avoidance of adverse health states. With one variant of this the willingness-to-pay for a reduction in the risk of death is used to infer the value of life. Thus if people will pay $200 for a 1 in 100,000 reduction in the chance of death, then it is inferred that the value of a life is $200 x 100,000 = $20 million).

Cost Effectiveness Analysis: The chief problem with CBA is that it requires the value of lives saved by a medical program to be measured in dollars and, the techniques used, including the willingness-to-pay, are not fully satisfactory (Richardson 1999). Cost Effectiveness Analysis (CEA) evolved as a method for avoiding this problem. Benefits are measured in natural units such as lives saved or life years gained and the task of the economist is to estimate the cost per unit of outcome achieved – the cost per life year or the cost per life saved. This does not permit a direct comparison of costs and benefits but allows programs to be ranked in order of their 'desirability' with, once again, the caveat that nothing else is of importance.

Cost Utility Analysis: CUA is a sub-set of CEA in which the value of life years is weighted by an index of the quality of life or, more accurately, by an index of the 'utility' (the strength of a person's preference) of the health state. Quality – utility – weights are normally derived from a representative sample of the public.

Limitations and Unresolved Issues

CBA verses CEA: While CEA may have evolved to avoid the practical and conceptual problems associated with the monetising of the value of life, it is now clearly recognised that the two measures of outcome are conceptually different. The willingness-to-pay techniques employed in CBA are reflecting preferences – utilities associated with different outcomes. CEA is measuring units of outcomes per sé. As the willingness-to-pay is income related, the ranking of projects by CBA may differ from the ranking achieved by CEA. Which criteria to employ – preferences or health outcome – cannot be determined by technical economic analysis. Orthodox economic theory assumes that benefits are based upon preferences – a view described as 'welfarism'. This does not, however, imply that preferences must always be the basis for social judgements and others have argued that health outcome per sé, as employed in CEA, should be the criterion of value – a view referred to as 'extra-welfarism'. It is clearly a matter of social choice whether the values embodied in economic evaluation should be welfarist or extra-welfarist. Public policy and, more particularly, the requirements of the Pharmaceutical Benefits Advisory Committee are explicitly extra-welfarist. It is, however, possible that policy could be modified as health outcome is traded off against the welfarist objective of satisfying preferences if these were in conflict with health outcome.

Perspective, Costs and the Decision Rule: In principle an economic evaluation could be carried out from several perspectives; that is, 'costs' and 'benefits' could be calculated to include the costs and benefits to different groups in society. In particular, benefits could be limited to those obtained by patients or expanded to include family, friends and the community at large who may be concerned with the health state of its citizens. Costs may be limited to those borne by the funding body or government; or expanded to those borne by the entire community.

Most government bodies including the PBAC advocate the adoption of this latter 'societal perspective' and consequently require the inclusion of all costs. However this leads to a problem when there is a fixed budget as shown below.

$$\begin{aligned} \text{Cost} &= C_{gov} + C_{pat} \\ &= \text{Cost to Government} \\ &+ \text{Cost to patient} \end{aligned}$$

The usual textbook criterion for maximising social benefit is to minimise the cost per unit of outcome, for example the quality adjusted life year (QALY). This criteria does not, however, lead to maximum social benefits if the criterion is used by a government agency whose expenditures are limited by a predetermined budget. This is illustrated below in the example in Table 1.

Table 1 Maximising Social Benefits Subject to a Budget Constraint

Example:		C_{gov}	C_{pte}	Total Cost	Benefit/ Cost	Gov. Buys	Net Benefit
Gov Budget = $100	P_A	1	99	100	10:1	100	90,000
Benefit/Unit: $P_A = P_B = 1{,}000$	P_B	10	0	10	100:1	10	9,990

In sum, when one of two possible projects has a lower total cost but higher cost to government, less of this program can be purchased from a fixed budget and the criterion for maximising social benefits should be revised to the decision rule: 'minimise' the ratio of budgetary expenditure to net social benefit – QALYs less total cost. This indicates how the maximum possible net benefit can be 'purchased' by the government. Unfortunately, this reintroduces the problem that cost utility analysis does not provide a monetary evaluation of QALYs and, consequently, the value of the denominator of this ratio cannot be calculated. The solution is to either monetise QALYs or, less rigorously, to 'take government budgetary expenditures into account'. As the former solution is highly problematical, it is the second that must be adopted at present.

Allocative Efficiency and the Evaluation Framework: In practice the scope of most economic evaluations is limited. In the case of the PBAC requirements, new drugs are compared with drugs already in use. In principle, this could lead to the adoption of a new and superior drug where previously drug therapy was 'cost ineffective' – too costly relative to the benefits obtained. This could result in the subsidisation of the new drug which, in turn, could lead to its widespread adoption and substitution for a superior non-drug therapy which was not subsidised. In principle, the subsidy should be applied to only the most cost effective intervention when all possible interventions both in and out of the health sectors are taken into account. In practice, this could entail such a broad set of options that this first best solution would be impractical. A variety of 'frameworks' for disease based evaluation protocols are currently being discussed and developed and their adoption could, potentially, overcome this problem.

Indirect Benefits: In principle, the health gain arising from an intervention should be compared with the net loss of resources arising from the program. These include both the direct costs – the costs of hospital and medical care etc – and any other change in resource use. This could be associated with the patient's loss of time and, consequently, contribution to the economy whilst undergoing a treatment or, conversely, the gain to the economy arising from the return to work permitted by a successful outcome. For this reason, it is commonly argued, that these 'indirect benefits' should be included in an economic evaluation as the resources gained for the GDP are no different from the resources lost because of the operation of the health program.

Two issues are associated with indirect benefits. The first, and the subject of recent controversy in the literature, is the identification of the relevant indirect benefits. While orthodox economics has commonly advocated the 'human capital' approach to measurement – the equating of indirect benefits with the full value of a person's wages – Dutch economists have recently argued for the adoption of the 'friction approach' which takes account of the fact that the loss of a person to the workforce will result in the employment of a substitute worker so that the net loss is restricted to

the period of adjustment. In principle, this latter approach should be preferred despite the practical measurement problems it encounters.

More recently, Olsen and Richardson (1999) have argued that the value of indirect benefits may vary between countries and, in the limiting case, be zero. This is because indirect benefits must necessarily vary in direct proportion to the 'productivity' of the patient and, when this is limited to employment benefits, to their contribution to the GDP. This would imply a systematic preference for programs which benefited high income earners and, if this were unacceptable, the indirect benefits may be 'socially irrelevant benefits'; that is, because of the equity implications an explicit judgement may be made that such benefits should not be included in economic evaluation.

Justice-based Considerations

As reflected in Figure 2 it has always been recognised that economic evaluation should acknowledge, in one form or other, the importance of equity and distribution. It has not, however, been particularly clear what is implied by these rather general terms[1]. More recent work has explored this issue empirically. Some of the results are summarised below:

Severity: Survey respondents in Norway, Australia, Spain and the USA have uniformly indicated that where two programs lead to the same health *improvements* as judged by the recipient patients, there should be a priority given to the program which benefits patients in the more severe initial health state. Economists have commonly argued that need per sé should not be a criterion for program evaluation and that only the value of health improvement is of importance. The recent results suggest that giving some priority to patients with the greatest need per se may be consistent with social values.

Rule of Rescue: It has often been argued that in the context of a crisis it is not possible to discriminate on the basis of economic costs; that this would require clinicians and emergency health workers to behaviour 'contrary to their humanity'. While this issue has been subject to relatively little empirical enquiry, it would suggest that priority could be given to emergency procedures even when these are cost ineffective and if there are no satisfactory alternative procedures. The Rule of Rescue could be seen as a context specific application of the importance of severity. That is, even if procedures are cost ineffective there may be value in 'being seen to do something' or in the preservation of hope, even when this is fairly small.

Certainty: In the USA, Ubel has found that survey respondents place disproportionate importance upon the extension of service coverage to the entire population even when this implies the guarantee of services to groups where the care will be cost ineffective. Guaranteed coverage eliminates the anxiety associated with the risk of non treatment.

Hope: It has similarly been suggested that where any effective treatment is excessively costly the provision of some care, *albeit* to an arbitrary sub-group of the total, may be desirable as it preserves the hope of treatment at some future time.

[1] The scope of the issues in most discussions of this topic has been very narrow and possibly reflects the limited connotations of the word 'equity'. While, in principle, equity could be equated with any issue of social justice, in practice it appears to have been primarily associated with 'access to', and the 'equal distribution' of health benefits. There is no 'in principle' reason for such a limitation. It merely reflects the fact that we are commonly 'prisoners of language'.

Anticipation: Cost effectiveness analysis normally includes benefits arising from actual or realised health states. It is possible that benefits may arise from the process of care. Certain procedures may give greater reassurance to patients, minimise concerns with respect to risk per sé and reduce the possibility of subsequent regret. A national health scheme, however, need not include benefits of this sort if, as suggested by pilot surveys carried out by the present author, the population does not wish to pay for these benefits. The generality of these preliminary results is not known and it is possible that some priority may be given in cases where the effects of anticipation are particularly strong.

Personal Characteristics: Well conducted studies in Australia, the UK and the USA have found that populations support the use of age weights; that is, attaching different importance to health gains according to the age of the beneficiaries. This is independent from the effect of age already incorporated in cost effectiveness analysis as a result of the reduction in life expectancy as people age. Such weights have already been incorporated in the Disability Adjusted Life Year (DALY). Suggestive but inconclusive evidence supports the differential treatment of benefits to patients in different socio economic classes and different treatment of smokers and non smokers in smoking related interventions.

Natural Versus Treatment Effects: Economic evaluation considers consequences but not the reason for these consequences. It is possible, in principle, that a different weight would be attached to an adverse health state when it was a result of the natural progression of a disease and when it was the adverse consequence of the intervention. The former case may be considered unfortunate but, in some sense, 'inevitable'. The latter case may be associated with feelings of anger or regret. This issue has not been investigated empirically.

Conclusions

The general conclusion of this paper is highly unsurprising: economists do not have all the answers yet and maybe never. There is a gap between theory and practice and, in particular, issues associated with social values and social justice have been poorly developed by economists. Table 2 summarises these issues. The general conclusion here should not be equated with scepticism about the value of economic evaluation. The issues discussed constitute grounds for qualifying and not for rejecting economic analyses. Many of the issues here will be irrelevant in many studies and their quantitative importance may be peripheral in other cases. That is, the results derived from many economic evaluations will quantitatively dominate the final decision. The important conclusion, however, is that there are numerous bases for rejecting the 'league table' approach in which priority is assigned in direct proportion to a cost benefit ratio where the constituent costs and benefits do not include all of the factors relevant to social welfare.

Table 2 Summary: Where Discretion may Cause Deviation from a League Table Ranking

Topic	Issue/Reason for Discretion
1. Welfarism vs Extra Welfarism	PBAC requires 'extra welfarist' focus on health gain. Trade-offs are possible with 'preferences'
2. Budgetary vs Societal Costs	A semi fixed budget requires consideration of budgetary costs
3. Breadth of Options Considered	A subsidy to a drug may discourage use of an unsubsidised non-drug alternative (exercise, diet)
4. Indirect Benefits	Effects on Employment, GDP and tax revenue are ignored by the PBAC Inclusion is justified if subject to measurement and equity caveats
5. 'Justice Based' social values	
(a) Induced Harm	Greater weight may be given to health states caused by therapy than 'natural' adverse health states
(b) Age** Weights	Defensible to discriminate by age (in addition to length of expected life)
(c) Social Weights	Defensible to discriminate by (—in favour of—) some social-ethnic groups (low SES; Koories, etc).
(d) Distribution*	Greater weight may be given where benefits more evenly distributed
(e) Severity**	An equal gain (as measured by the patient) may be more valued (by society) when the initial state is more severe
6. Realised vs Anticipated Health	'Realised' health states are currently measured. A national health scheme may/may not be concerned with process effects and anticipation utilities (fear, hope, anticipated regret, disutility of risk, etc)
7. Rule of Rescue	'Cost ineffective' therapies may be justified in the context of a crisis
8. Health Potential (Double Jeopardy)	'Benefits' be adjusted (upwards) when health gain is limited due to a pre existing long term health condition (quadriplegia; chronic illness)

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