Addressing risk preferences in cost-effectiveness analyses

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**Abstract:** Cost-effectiveness analysis is a form of economic evaluation that compares the costs and effectiveness of health interventions, where effectiveness is measured in a single scale. Despite the growth in the popularity of cost-effectiveness analysis, very few cost-effectiveness analyses adequately measure and account for uncertainty. In the health economics literature, two schools of thought are emerging. The first takes a statistical approach to uncertainty by focusing on the likelihood that a decision making error will be made. The second approach applies and develops economic theories of risk preference that consider the welfare implications for a patient when they are presented with interventions that have uncertain health outcomes. Cost-effectiveness analyses need to account for risk preferences if they claim to be increasing patient welfare.

**Keywords:** cost effectiveness analysis, uncertainty, risk aversion

**Introduction**

Cost-effectiveness analysis (CEA) is a type of economic evaluation that looks at the trade-off between scarce resources (costs) and health benefits (effectiveness) for particular health interventions, where health benefits are measured on a single scale. While there is flexibility in the application of CEA, a number of guidelines for the use of CEA have been widely publicised in medicine (eg Drummond et al 1997, p 96–158), and national guidelines have been established in several countries. Most of the work on CEA methods has been procedurally based, although several fundamental questions linger concerning the theoretical foundations of CEA. One of the areas currently being discussed in the health economics literature is how to modify CEA to incorporate uncertainty into the decision making framework. This brief note gives a general overview of why a measure of uncertainty should be included into the CEA framework, discusses the various methods proposed for such analysis, and finally explains how economic theory on risk preferences may be used to guide future modifications to CEA.

**What is wrong with CEA?**

Over the past two decades, the number of health intervention CEA has climbed steadily (Elixhauser et al 1998, p MS4–MS5), especially in countries with national health programmes. While CEA has influenced the adoption and implementation of new health care technologies, it appears not to be the sole basis for such decisions. Given that health policy decisions are affected by factors outside the scope of CEA methods, one may conclude that the current methods are inadequate. An ‘orthodox’ response would be that economists should only be concerned with clear-cut questions such as (technical) efficiency. As such, addressing differences between analytical results and actual decisions should be treated as an activity beyond the scope of the economic discipline. Health economists, however, have always been interested in discussing what should be the goals of health care (Blaug 1998, p S63–S64; Hurley 2000, p 59–66). This focus on the objectives of health policy has led to a broader perspective of outcomes in the economic evaluation of health care. To these ends, health economists are now incorporating quality of life, equity and process considerations into evaluation methodologies (Ryan 1999, p 535; Lindholm et al 1998, p 808; Mooney 1994, p 151–4). Recent efforts to refine outcome measures have placed considerable attention on the role that uncertainty plays in...
evaluation, particularly in CEA. It is these advances that are the focus of the remainder of this paper.

**How does uncertainty affect CEA?**

It is important to stress that there is a general division in the medical decision making and health economics literature regarding how to account for uncertainty in CEA. To highlight this point, consider an example from Bridges et al (2002, p 49). Assume that a certain (fixed) budget can be spent on a particular intervention. The effectiveness of this intervention will vary across individuals, and thus the effectiveness for a given population can be considered random. While some authors have proposed that a portion of this variation in effectiveness can be explained by individual characteristics (Birch 1997, p 555), for the purpose of this analysis we will assume that all variations are purely random. In addition, Sculpher and Gafni (2001, p 318–9) discuss the importance of variations in consumer tastes, and while this is an important issue in economic evaluation, we will also assume that there are no major variations in consumer tastes and that a suitable aggregation mechanism can be used to illicit social preferences.

Returning to our example, let us denote the effectiveness of the intervention for a representative individual as a random variable, $E$. Employing CEA as a guide to decision making, we would search for the intervention that yields the largest $E$ possible, assuming resources are fixed. One can call $E$ the ‘standardised effectiveness ratio’, as it is standardised for costs.

One branch of the literature on CEA and uncertainty focuses on the probability that the average effectiveness ($\bar{E}$) is higher than some critical point, say $E^*$. This approach takes a statistical approach to uncertainty, where uncertainty affects the likelihood that a decision making error will be made. Most of the work in this area is centred on techniques to estimate univariate (or multivariate) confidence intervals to reflect parameter uncertainty (eg Chaudhary and Sterns 1996, p 448–51; O’Brien et al 1994, p 158–60; Wakker and Klaassen 1995, p 375–7; Briggs et al 1997, p 328–9). For example, a simple (95 percent) confidence interval for a random $E$ can be given as: $\bar{E} \pm 1.96 \times SE(E)$, where $SE(E)$ is the standard error for $E$ and is a measure of the uncertainty involved in the estimation of $\bar{E}$.

The second arm of the literature takes a welfare theory approach to analysing the affects of uncertainty. This literature extends upon the statistical concerns discussed above to examine the welfare effects of presenting individuals with uncertain outcomes. Thus, patient welfare will be affected not only from the average effectiveness ($\bar{E}$) – often referred to as the ‘return on the intervention’ – but also from the uncertainty in that effectiveness, that is, $SE(E)$. If patients are risk averse, ie they dislike uncertainty, then a larger effectiveness on average will increase their utility, while a greater uncertainty in effectiveness will decrease their utility. More generally, this approach focuses on patient (or societal) welfare, recognising that the entire distribution of outcomes that may arise as a result of an intervention affects the wellbeing of all of those who may consume it. Much like decisions to invest in the stock market, decisions to use health interventions should involve a trade-off between return and uncertainty.

**Risk aversion: a theoretical justification**

Methods that solely focus on the means of effectiveness (or the average cost-effectiveness ratio) implicitly enforce a risk neutral perspective, assuming that individuals have no distaste for uncertainty and only care about average outcomes. The justification for neutrality often hinges on a result called the Arrow-Lind theorem, which argues that social decision makers should be treated as risk neutral (Arrow and Lind 1970, p 355–6). In the health context, this argument is tantamount to assuming that the uncertainty associated with a health intervention is spread across a large population such that no individual bears a significant portion of the financial burden associated with the intervention (Meltzer 2001, p 115–6). However, in countries where health insurance is obtained in the private market, risk spreading is limited, suggesting that the financial burden borne by individuals is not trivial. Moreover, even in countries with nationalised health systems, where the costs of medical care are spread across all of its citizens, individual health outcomes are not diversifiable. Citizens cannot fully share the pain, suffering, and other burdens resulting from their illnesses. Thus, the conditions necessary for the Arrow-Lind theorem and the assumption of risk neutrality to hold are not generally obtained in the health care context (Ben-Zion and Gafni 1983, p 162–4; Graff Zivin 2001, p 500–2). Furthermore, many of the rules that govern the actual provision and protection of health suggest that risk aversion is an important principle guiding decision making, with the concept of nonmaleficence being prominent in medical ethics (Beauchamp and Childress 1994, p 193–4).
Even if social neutrality were theoretically justified, insurers, physicians and patients make most health care decisions at sub-societal levels. Insurers deliberate over which interventions to cover and physicians and patients jointly decide which care paths to pursue. For these decision makers, risk aversion is the norm. If CEA does not incorporate this preference, then it is not surprising that the choices made by decision makers do not conform to cost-effectiveness evidence. Given the predominance of decision making at these levels, CEA, which is meant to guide coverage and treatment decisions, must attend to sub-societal concerns about uncertainty. Indeed, such concerns were identified as an area of important future research in the medical decision making literature over a decade ago (Deber and Goel 1990, p 193–4).

**CEA and risk preferences**

To date, only a few cost effectiveness studies have explicitly addressed risk preferences in their analyses. Most studies have assumed a risk-neutral decision maker. These papers, invoking Arrow-Lind type arguments about diversified risk, have generally concluded that the ratio of average costs to average benefits is sufficient to characterise the cost-effectiveness ratio (Claxton 1999, p 347–50; Meltzer 2001, p 116). As discussed above, however, risks in this context are often not fully diversifiable, casting doubt on the theoretical validity of assuming risk neutrality. Moreover, there is extensive economic literature that provides empirical support for preferences consistent with risk aversion (eg Friend and Blume 1975, p 906–15; Hansen and Singleton 1983, p 261–4), as well as literature that explicitly examines uncertainty over health outcomes (Viscusi and Evans 1990, p 353–4).

The theoretical studies that explicitly address CEA when decision makers are risk-averse are small, but a new body of literature has started to emerge. The earliest work was conducted by Garber and Phelps (1997, p 1–31), who developed a guide to resource allocation using cost-effectiveness criteria based on standard expected utility, the workhorse for modelling microeconomic decision making under uncertainty. They found that optimal cost-effectiveness ratios, above which an intervention should not be undertaken, depend on a host of *individual* characteristics, including the degree to which an individual is risk averse. Sensitivity analyses over a wide range of parameter values demonstrated the particular importance of risk aversion in determining the desirability of an intervention.

Graff Zivin (2001, p 500–2) expanded the focus of this work to examine the role of risk aversion at a societal level. Graff Zivin’s work demonstrated that even societal decisions based on CEA might lead to a decrease in social welfare if risk preference was not adequately incorporated into the decision making criterion. If, for example, a new intervention increases both average effectiveness (return) and uncertainty (risk), standard CEA would advocate adopting the new intervention, although for some levels of risk aversion the decision would decrease societal welfare. Likewise, if a new intervention had less risk and return than the status quo, traditional CEA would reject the new intervention, even though it might be associated with an increase in social welfare. As a result, Graff Zivin (2001, p 506) proposes reporting the cut-off social rate of risk aversion that would be sufficient to change the preferred treatment strategy as identified by traditional CEA analysis. Such reporting would allow decision makers to quickly assess whether the relative likelihood that the population’s disutility from risk is large enough to overturn the policy decisions implied by the traditional approach.

A more general approach to evaluation is to consider all funded interventions jointly in a portfolio. O’Brien and Sculpher (2000, p 461–3), using portfolio management methods from the financial economics literature, proposed such a model as a guide for health care decision making. Under this approach, individual interventions are not viewed in isolation, but rather as part of a ‘portfolio’ of interventions constructed to maximise total benefits per dollar spent on health care interventions. Here benefits not only stem from the intervention increases both average effectiveness ($E$) of the portfolio of interventions, but from decreasing the uncertainty associated with it, that is, $SE(E)$. Thus, decision makers allocate their interventions to construct a portfolio that maximises return and minimises risk. To make such a calculation, one needs to know the individual intervention’s effectiveness and uncertainty, as well as how each intervention affects the effectiveness and uncertainty of the portfolio as a whole.

Bridges et al (2002, p 49) builds upon this concept by allowing for *synergies* between interventions. Synergies are defined as the positive, or negative, effects of using combinations of interventions as compared to the linear sum of the individual interventions. For example, consider that the standardised effectiveness for one intervention is $E'$ and for another (distinct) intervention is $E''$. If we were then to consider a portfolio consisting of the two interventions with a fraction $p$ of the budget spent on the first and the remaining $(1−p)$ on the second, then a first approximation of the
standardised effectiveness of the portfolio would simply be $pE' + (1-p)E''$. Bridges et al (2002, p 49), however, show that a more general approach would allow for the two interventions to interact – positively or negatively – and thus the standardised effects may be $pE' + (1-p)E'' + S$, where $S$ denotes this level of interaction or synergy.

This analysis demonstrates that the choice of the right interventions for the portfolio needs more information than just the individual effectiveness for each individual, but the joint effects. The implications from such a model are immense. A social decision maker not only needs to consider all possible interventions in terms of risk and return, but consider all possible combinations of all interventions. Given the near impossibility of such a task, Sendi et al (2002, p 25–7) propose a ‘second best’ alternative that computationally makes improvements in spending a global budget. This method fails to be ‘first best’ in that it cannot find the optimal portfolio on which to spend the global budget instantaneously, but rather relies on an iterative method of improvements that converges to the ‘first best’ outcome. These iterations rely on the health care planner considering the opportunity costs of new health expenditures in terms of their effects on the overall return and uncertainty of the health budget. While the Sendi et al method does not specifically address the return/uncertainty trade-off in a welfare sense, it could easily be modified to do so.

**Conclusion**

Health economists should no longer use simple methods based on averages for CEA. Given the variety of methods to account for risk, however, a general consensus concerning a single appropriate method will be difficult to reach. It is widely known that economists have never really been good at consensus building, preferring instead to encourage lively debate. The debate in macroeconomics over the effects of fiscal and monetary policy is a classic example. Such disagreements are not limited to theory or philosophy. Methodological discordance abounds as well. For example, economists have developed numerous regression methods for isolating causation, but the optimal choice of methods depends mainly on the specific circumstance of the analysis.

So why do we need a standard method for CEA? The answer is that two critical and unavoidable factors demand a single uniform method of CEA. First, the purpose of CEA is not an evaluation of a single intervention for a specific disease-type, but to compare across all interventions and disease-types to set priorities for funding – a single methodology is required to optimise this process. Second, given that CEA will be used to affect clinical behaviour, either implicitly by influencing individual decision makers or explicitly, by advising global funding decisions, we must present a parsimonious methodology to maintain credibility. This leaves the health economists somewhere between a rock and a hard place – should we continue to work with parsimonious, yet theoretically inferior methods, or should we increase our efforts in developing better evaluation tools?

Like any form of research and development, efforts to develop a better evaluation tool in health economics will have opportunity costs. These costs will not only include the direct time and effort by the profession, but the indirect effects (or transition costs) felt by those who use economic evaluations in their decision making. These short-term costs, however, must be weighed against the potential future gains from more productive and socially beneficial economic evaluations. The theoretical research discussed above may be an indication that this methodological revolution in the economic evaluation of health care interventions has already begun.

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**References**


