

‘HEALTH ECONOMICS’ AND THE EVOLUTION OF ECONOMIC EVALUATION OF HEALTH TECHNOLOGIES

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1. INTRODUCTION

From its inception, Health Economics (HEC) has had a particular focus on the methods of economic evaluation as applied to health care and health policy. Since 1992, HEC has published over 250 research papers and over 70 comments, letters and editorials on economic evaluation of health technologies and policies—an average of two contributions per issue. Almost equal numbers of articles have been published in each decade of the journal’s existence, indicating a decline in the proportion of articles on evaluation in the journal, as the annual number of issues (and the size of each issue) has increased over time. This does not reflect a decline in interest in the topic, rather an expansion in the scope of the journal. In fact, there has been a major increase in international interest, which has led to the creation of several new specialist journals in the field of health technology assessment.

In its early days, HEC provided an outlet for the growing number of papers on the methods of economic evaluation in health care which could not easily find a place in the existing specialist HEC journals, nor in more general applied economics journals. HEC did not publish evaluation papers purely as a record of their empirical results. Such papers were increasingly being placed in clinical journals, as their results were intended to influence the delivery of health care, and now find outlets in the newer technology assessment journals. HEC concentrated on papers, which identified advances in evaluation methods and improvements in the data sources available for use in evaluations.

In the 1990s, the first decision-making systems formally requesting submission of cost-effectiveness data to support applications for the reimbursement of drugs and other technologies were introduced in Australia and Canada, followed in 1999 by the creation of the National Institute for Clinical Excellence (NICE) in England and Wales. This stimulated further interest in the methods of economic analysis, with an additional focus on the suitability of the methods for practical application in a formal decision-making process. This latter aspect came to the fore as the reimbursement agencies tried to develop evaluation guidelines to impose some consistency on the information produced for each technology appraisal.

This paper will not attempt to review systematically 20 years of literature on economic evaluation in health care. There is neither time nor space to do that. What it will do is provide a more selective review of the influence of contributions to the journal on the development of economic evaluation, in terms of its relationship to economic theory, advances in analytical methods and the practical considerations in adapting economic evaluation for use in a routine decision-making context. In the following sections, I shall discuss examples from each of these three areas.

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2. CONCEPTUAL ISSUES

2.1. Extra-welfarism

Although there was general agreement on the broad principles of economic evaluation to be applied to health care interventions and technologies by the early 1990s (particularly in the UK), there remained many areas of significant disagreement. The most fundamental division was between the welfarists and the extra-welfarists regarding the appropriate analytical framework and outcome measures to be used in evaluation. This was in some ways a continuation of the debates in the cost-benefit literature of the 1960s and 1970s between the 'Pareto' and 'decision-making' schools. In the absence of market values, the former advocated the valuation of effects using proxies for individual willingness-to-pay (WTP), whereas the latter emphasised the role of the decision-making body in determining the appropriate social values for benefits and costs (Mishan, 1971; Sugden and Williams, 1978). HEC has published contributions from both perspectives, for example, Hutton and Maynard (2000), Birch and Gafni (2002) and Chalkidou *et al.* (2008). Looking back, there are many points of agreement between the two groups, and the differences seem to be more in terms of the practical significance of conceptual weaknesses in the health economic evaluation framework, than over the existence of the weaknesses themselves.

Both sides accept that the quality-adjusted life-year is not a perfect measure of utility in the economic sense and that, in principle, WTP would be better. However, opinions differ over whether quality-adjusted life-years (QALYs) are a sufficiently good approximation of the relevant benefits and whether hypothetical WTP values are a sound basis for social decision making. There also is general acceptance that the empirical justification for the NICE cost-effectiveness threshold range of £20 000–30 000 is weak. Disagreement arises over whether the use of a threshold test for new technologies is the correct analytical approach, regardless of whether the threshold is an accurate reflection of social valuation of health benefits.

The extra-welfarist position is that the use of the best available measure of health benefits (QALYs) in a marginal comparison of benefit per £ spent is the correct approach in a budget-constrained health system. Validating the cost-effectiveness threshold and improving the outcome measures are empirical questions. The welfarists argue that an attempt must be made to measure utility in its fullest sense and that societal benefits from the health budget should be maximised in total (i.e. including the externalities), not just in terms of health. They argue that this model can explain 'unhealthy' choices by individuals, who gain other forms of utility from activities that damage their health, and that some forms of public health intervention are doomed to fail. Acceptance of the rational consumer model in health services opens up much wider issues about the rationale for state intervention in the sector, which in other contexts, many welfarist authors seem to favour. As well as conceptual problems and the empirical issue of determination of the appropriate cost-effectiveness threshold, this debate also has raised the practical issue that the opportunity cost of the budget constraint may vary between different parts of a national health system.

2.2. Discounting

Another important debate, which featured in HEC and which is still ongoing, relates to discounting of future health benefits and costs. An early paper by two economists from the UK Department of Health challenged the then orthodox view that costs and benefits in an evaluation should be discounted at the same rate (Parsonage and Neuburger, 1992). Subsequent papers challenged but did not totally reject their position, for example, Cairns (1992) and Van Hout (1998). The result was that, with Treasury backing, the first economic evaluation guidance issued by NICE recommended the use of discount rates 6% for costs and 1.5% for benefits (NICE, 2001). This was contrary to guidance produced in other countries, which favoured equal discount rates of between 3% and 5% for costs and benefits.

The debate developed with important contributions to HEC from Gravelle and Smith (2001); Claxton *et al.* (2006) and Gravelle *et al.* (2007). A new consensus began to emerge that, although there were arguments that society should discount both future costs and benefits, it was not clear that it should be at the same rate. However,

the point also was made that in a health system funded by a constrained but ear-marked annual budget opportunity costs should be measured in terms of health gained or lost so consistent discounting was in order. NICE accepted this argument and changed its guidance to 3.5% for both costs and benefits (NICE, 2004), just as the academic debate was beginning to support their original position (if not their choice of absolute values). As I write this, I understand that a change back to differential discounting is being implemented.

2.3. Outcome measures

In parallel with the ongoing debate between the welfarists and extra-welfarists, HEC received many contributions on the development of outcome measures for use in economic evaluations. Acceptance and application of the QALY became widespread in the 1990s, and its further use was encouraged by the publication of the UK EQ-5D health state values derived in the Measurement and Valuation of Health study (Dolan *et al.*, 1996). Several papers were published challenging the unthinking use of QALY measures, for example, Gerard and Mooney (1993) and Nord (1994). Other approaches to the derivation of utilities, such as SF-6D, were frequently discussed and compared with QALYs (Brazier *et al.*, 2004).

Major coverage was given to the development and application of WTP studies and discrete choice experiments (DCEs) as alternative sources of outcome measures. In addition to reports of individual experiments and empirical studies, several articles were published setting out the pros and cons of the use of DCE, for example, de Bekker-Grob *et al.* (2010) and Lancsar and Savage (2004).

3. UNCERTAINTY AND STATISTICAL METHODS

Another area in which much progress has been made in the last 20 years is in the characterisation, measurement and communication of the uncertainty surrounding the results of economic evaluations. HEC has published a series of influential contributions on these issues beginning with definitive reviews of possible approaches and past practice (Briggs *et al.*, 1994; Briggs and Sculpher, 1995). As data from clinical trials were increasingly used in evaluations, probabilistic approaches to sensitivity analysis began to be used. The representation of uncertainty through cost-effectiveness acceptability curves was introduced in an early paper by Van Hout *et al.* (1994). Subsequent papers have provided practical assessment of the use of PSA and CEACs in decision making (Fenwick *et al.*, 2001; Claxton *et al.*, 2005).

The approaches to the analysis of uncertainty outlined above the need data on the distribution of parameter values. Randomised clinical trials (RCTs) offer data in this form and economists using RCTs as a source of data on the clinical efficacy of technologies, also saw trials as a vehicle for collecting patient outcome and resource use data. Although undoubtedly better than many data sources used in early economic evaluations, clinical trials, particularly those relating to pharmaceuticals, bring their own problems.

One such problem is the relevance of results based on pooled data from international clinical trials. Can the aggregated data be applied in each country involved in the trial, or is further analysis needed to assess the impact of technologies in an individual country or health system? Several authors contributed to this debate, proposing econometric approaches to control for potential biases in the pooled data. Notable contributions were made by Willke *et al.* (1998), Willan *et al.* (2004), Hoch *et al.* (2002), Claxton and Posnett (1996) and Sculpher *et al.* (2006).

Another challenge to the relevance of clinical trial data is the potential variation of resource use in the trial situation from that in routine clinical practice (the latter being deemed more relevant to the assessment of the ultimate cost-effectiveness of a technology). Even when a trial is considered relevant to routine care, there remains the issue of missing data and the potential biases it may introduce (Briggs *et al.*, 2003). These issues were important in the debate over the best analytical framework for evaluations of technologies discussed in the next section.

4. PRACTICAL APPLICATION OF ANALYTICAL FRAMEWORKS

Just as working more closely with clinical researchers and biostatisticians to derive outcome data from clinical trials led economists to re-think the rigour of the resource use data they were accustomed to using, the increasing use of health economic evaluation to inform service delivery decisions forced clinicians to recognise the importance of the opportunity costs of healthcare resource use. The use of cost-effectiveness analysis in routine decision-making contexts also led to consideration of the relative desirability and feasibility of trial-based and model-based economic analyses.

Although the early debates often presented clinical trials and decision models as alternative frameworks within which to conduct economic evaluations, by the end of the 1990s, it became accepted that they were complements rather than substitutes. Two important contributions to this debate were published in HEC. Sheldon (1996) highlighted the poor quality of clinical outcome information available for use in models and the failure of authors to explore fully in sensitivity analysis the uncertainty resulting from unreliable data. In a subsequent editorial, Buxton *et al.* (1997) addressed the issues of rigour and relevance in the use of modelling to inform decisions on the utilisation of health technologies. Their conclusion was that some element of modelling was always necessary, even when data were available from the highest quality empirical studies, to make that data relevant to the question being addressed. Very often, it is necessary to extrapolate beyond the duration of a trial when long-term outcomes are important. This is particularly the case when trials use intermediate clinical outcomes and final patient outcomes and resource cost are expected to occur well into the future. If economic evaluation is being used to guide decisions on the reimbursement of new technologies at the time of their launch, by definition, true effectiveness data will not be available for that technology relative to comparators. Decisions have to be made, and formal analysis in a decision-modelling framework is the best way to make use of the available data.

The importance of the short and focussed contributions facilitated by Health Economic Letters is illustrated by two contributions to this debate. Donaldson *et al.* (1996) pointed out that it is not sensible to plan to compare technologies solely on their economic impact (i.e. cost-minimisation analysis), as complete clinical equivalence is seldom demonstrated empirically. Dowie (1997) asserted that clinical trials are not purely clinical—they should be used to collect socially relevant outcomes and economic data. A clinical trial or an economic analysis alone is a partial evaluation, an insufficient basis for a social decision. We should think in terms of the single activity of ‘evaluation’, incorporating all the relevant information.

As these debates developed the interaction between concepts, empirical application and the practical needs of decision-makers became apparent. For example, the successive versions of NICE technology appraisal methods guidance record expectations with regard to the methods of analysis to be applied, the types of data preferred and the analytical framework within which the results (and the uncertainty surrounding them) are to be presented. Improvements in the availability of data and techniques of analysis raised expectations with regard to the rigour of economic evaluations.

5. CONCLUSION

My selection of topics and papers for discussion is in no way intended to imply that the many other contributions to HEC on economic evaluation have been less useful and less important. The examples I have chosen are intended to illustrate some general points about the way HEC has contributed to the development of thinking in the field of economic evaluation, not to be a summary of that contribution.

The papers published in HEC, which have been highlighted here, are important for a variety of reasons. Of the conceptual papers, some are seminal contributions, which opened up new developments in methods; others are considered responses to seminal papers published elsewhere, whereas others are definitive accounts of the state of the art in particular topic areas at particular points in time. The last category is important as these contributions have not only marked the advance of debates for specialists but have provided valuable teaching

resources, not just for students of health economics but also for the large numbers of researchers and decision makers from other disciplines involved in the field of health technology evaluation. The success of health economists in influencing decision making on the adoption of health technologies has resulted partly from their ability to communicate their ideas in a manner which has been accessible to non-specialists.

One of the disadvantages of becoming influential in the practical world of health system decision making is that pressures can be brought to bear to adopt methods which are likely to suit the agenda of particular groups. As increasing numbers of countries claim to be basing decisions on the reimbursement of health technologies on some form of economic evaluation, health economists must beware of policy initiatives, which may undermine the value of using economic methods. Even in England and Wales, where NICE has evolved a transparent and rigorous approach to decision making with the economic evaluation framework at its core, 12 years of hard work have been put in jeopardy by hastily concocted proposals for a system of 'value-based pricing' (Department of Health, 2010). To maintain the influence of health economics, to paraphrase Robert Lowe (commenting on the extension of the franchise in Britain in 1868), 'we must continue to educate our masters'. HEC will continue to have an important role in that process.

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