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Addressing uncertainty in medical cost–effectiveness analysis Implications of expected utility maximization for methods to perform sensitivity analysis and the use of cost–effectiveness analysis to set priorities for medical research

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Abstract

This paper examines the objectives for performing sensitivity analysis in medical cost–effectiveness analysis and the implications of expected utility maximization for methods to perform such analyses. The analysis suggests specific approaches for optimal decision making under uncertainty and specifying such decisions for subgroups based on the ratio of expected costs to expected benefits, and for valuing research using value of information calculations. Though ideal value of information calculations may be difficult, certain approaches with less stringent data requirements may bound the value of information. These approaches suggest methods by which the vast cost–effectiveness literature may help inform priorities for medical research. © 2001 Elsevier Science B.V. All rights reserved.

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1. Introduction

Despite some recent slowing in the growth of health care costs in the US, health care costs have risen substantially over the past several decades and are likely to continue rising (Smith et al., 1998). This appears to be largely due to the growth of new technology (Fuchs, 1990; Newhouse, 1992). While improvements in health are highly valued (Cutler and Richardson, 1997; Murphy and Topel, 1998), evidence from diverse methodological perspectives suggests that many technologies may have little value at the margin (Eddy, 1990; Brook et al., 1983; McClellan et al., 1994). Cost–effectiveness analysis and other methods for medical technology assessment have arisen to attempt to address this important problem.

One of the main challenges faced by medical cost–effectiveness analysis has been the question of how to perform these analyses in the presence of uncertainty about the benefits and costs of medical interventions. The uncertainty of primary interest in this regard is uncertainty in population level outcomes, although uncertainty in outcomes at the individual level may be present simultaneously. This uncertainty in population level outcomes may result either from limited evidence from clinical trials or the need to extrapolate based on the results of clinical trials using decision analysis and its associated uncertainties in the structure and parameters of decision models. This uncertainty concerning the benefits and costs of medical interventions has motivated much interest in sensitivity analysis within medical cost–effectiveness analysis.

Yet though there have been many proposals about how to address uncertainty in cost–effectiveness analysis, there has been relatively little discussion of the objectives for performing sensitivity analysis. Without a clear understanding of these objectives, it is difficult to know by what criterion to assess the merits of the many alternative approaches to sensitivity analysis. Thus, the lack of clarity concerning the objectives for sensitivity analysis is an important reason for the continuing ambiguity about how to address uncertainty in cost–effectiveness analysis.

This paper attempts to identify the objectives for sensitivity analysis within cost–effectiveness analysis and to develop methods suited to reaching those objectives. The primary objectives of sensitivity analysis are argued to be: (1) to help a decision maker make the best decision in the presence of uncertainty, (2) to identify the sources of uncertainty to guide decisions for individuals or subgroups with characteristics that differ from a base case, and (3) to set priorities for the collection of additional information. This paper studies these problems by examining the implications of an expected utility maximization model for the optimal choice of medical interventions when there is uncertainty about the costs and benefits of those interventions. The results indicate that if the objective is to maximize expected utility given available information — as is implicit, for example, in the maximization of quality-adjusted life expectancy — and if financial risk is effectively diversified through either public or private insurance, then the optimal decision is determined by the ratio of the expected cost divided by the expected benefit. Other assumptions about preferences or insurance will yield other conclusions about how to account for uncertainty (Mullahy, 1997), but also would require different models for cost–effectiveness in the absence of uncertainty at the population level. These findings also have implications for sensitivity analyses done for other purposes. If the objective of sensitivity analysis is to guide decisions for subgroups that differ from the base case, then the ratio of expected