



Economic evaluation to inform health care decision-making: Promise, pitfalls and a proposal for an alternative path

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ABSTRACT

Health economic evaluation aims at providing information on the efficiency of interventions. Since the 1980s, there have been major developments in the field, especially in terms of methodologies. As the field has expanded and developed, methodologies have become increasingly sophisticated. In parallel, over the past decade, the conduct of economic evaluations has become more and more institutionalized with, among other things, the creation of the National Institute for Health and Clinical Excellence (NICE) in England and Wales and a growing number of health technology assessment (HTA) agencies around the world. Yet the literature has identified important barriers to the use of economic evaluation in decision-making, among them the difficulty of deciphering economic evaluation research. The way the field expanded has thus created a paradox: whereas economic evaluation is seen as an insightful tool for achieving efficiency in health care, its methodological developments have decreased decision-makers' capacity to use it. In this paper, based on a literature survey, we explore this shift by first analyzing how the field of economic evaluation has developed in recent years. Second, we discuss how economic evaluation information is perceived and used in decision-making. Third, we consider a possible direction for reconciling economic evaluation and decision-making. The originality of this article is that it not only highlights the increasing gap between the aim of economic evaluation and its effective use in decision-making but also proposes, based on existing methodologies, a competing approach to the currently dominant paradigm.

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Introduction

Health economic evaluation aims at providing information on the efficiency of interventions. Efficiency results when benefits are maximized and opportunity costs (i.e., the value of benefits forgone by choosing one particular allocation of scarce resources over another) minimized (Donaldson, Currie, & Mitton, 2002). Initially, economic evaluation was a method intended to help health care decision-makers make the best choices under conditions of uncertainty, conflicting objectives and resource constraints (Weinstein, 2006). However, it rapidly proved inadequate for setting health care priorities. For example, in 1990, the state of Oregon tried using economic evaluation for health care priority setting. To determine what services would be covered by Medicaid, the Health Services Commission used cost-utility ratios to rank services according to efficiency (Tengs et al., 1996); in

those results, life-saving interventions were ranked below less critical items such as headache treatment (Hadorn, 1991; Tengs et al., 1996). This experience clearly demonstrated the unacceptability of using efficiency as the only criterion to prioritize health care resource allocations (Hadorn, 1991, 1996; Pinkerton, Johnson-Masotti, Derse, & Layde, 2002; Tengs et al., 1996; Ubel, Loewenstein, Scanlon, & Kamlet, 1996). Since then, several authors have analyzed the philosophical and theoretical foundations of the utilitarian approach to economic evaluation in relation to access to care, emphasizing the inadequacy of considering only this value in priority setting (Domenighetti, 1998; Veatch, 1995; Williams, 1992; Williams & Cookson, 2006). Nevertheless, economic evaluation has become increasingly institutionalized with, among other things, the creation of the National Institute for Health and Clinical Excellence (NICE) in England and Wales and a growing number of health technology assessment (HTA) agencies around the world. The trend toward evidence-based decision-making reinforced the need to base resource allocation decisions on rational criteria, with effectiveness and efficiency being especially important. In fact, it is widely

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recognized that inefficient resource allocations have important consequences in terms of opportunity costs, e.g. reduced access to care for other patients. Furthermore, it is now recognized that other criteria, such as equity of access to care and the rule of rescue (Caro, 2009; Domenighetti, 1998; McKie & Richardson, 2003; Nord, Pinto, Richardson, Menzel, & Ubel, 1999) should be considered at the same time as efficiency of interventions.

Since the 1980s, there have been major developments in the field of economic evaluation, especially in terms of methodologies, which have become increasingly sophisticated. At the same time, the literature has identified important barriers to the use of economic evaluation in decision-making; the difficulty of deciphering economic evaluation research being one of them. The way the field has expanded has thus created a paradox: whereas economic evaluation is considered an insightful tool for achieving health care efficiency, its methodological developments have decreased decision-makers' capacity to use it.

In this paper, we explore this shift by first analyzing how the field of economic evaluation has developed in recent years, focusing particularly on trends in methodological sophistication. Then we examine how economic evaluation information is perceived and used in decision-making. Finally, we discuss an alternative path, based on different existing methodologies, for reconciling economic evaluation with decision-making. This analysis is based on a literature survey that documents the interfaces among currently recommended methodologies, as well as recent developments in economic evaluation and its use in decision-making. The analysis uses various published sources: 1) methodological guidelines and seminal textbooks for economic evaluation (CADTH, 2006; Drummond, Sculpher, Torrance, O'Brien, & Stoddart, 2005; NICE, 2008); 2) health economists' systematic reflections on bridging the gap between economic evaluation and decision-making; and 3) analyses of the use of economic evaluation in health care decision-making, including six systematic reviews.

Economic evaluation: current developments

Economic evaluation has evolved considerably over the past 20 years, with greater recognition and more resources, new research challenges, and more sophisticated techniques and methodologies. Guidelines and consensus-based recommendations for conducting, reporting, and appraising economic evaluations have become increasingly standardized. In this section, based on a literature survey and analysis of experts' views, we describe the methodological evolution of a field whose aim is to enhance the application of the efficiency criterion in the allocation of scarce health care resources. We discuss current economic evaluation practice, focusing on its methodological developments and ability to inform decision-making.

Types of economic evaluation

Economic evaluation provides information on the efficiency of interventions. It compares costs and consequences of at least two interventions, the one under study and a natural comparator. Several types of economic evaluation exist: cost-minimization analysis (CMA), to be used if the effects of both interventions are the same in nature and quantity; cost-effectiveness analysis (CEA), to be used if the effects are of the same nature but with different results in each of the compared interventions; cost-utility analysis (CUA), which is appropriate if the intervention affects both length and quality of life; and cost-benefit analysis (CBA), which looks at efficiency in resource allocation by comparing effects, which are assigned dollar values, to costs (CADTH, 2006; Drummond, Sculpher, et al., 2005). The NICE and the Canadian Agency for

Drugs and Technology in Health (CADTH) guidelines both recommend reporting results as incremental cost-effectiveness ratios (ICERs) (CADTH, 2006; NICE, 2008). The ICER is the ratio of the difference of costs over the difference of outcomes of the various alternatives (Drummond, Sculpher, et al., 2005).

Long-standing methodological issues and debates

While economic evaluation offers a seductive format (with one concise indicator) for decision-making, it is not a perfect instrument, and there are long-standing methodological issues and debates on various aspects such as: 1) variations, depending on the instrument used, in utility scores obtained for calculating quality-adjusted life years (QALYs), creating uncertainty around validity (Badia, Roset, & Herdman, 1999; Bleichrodt & Johannesson, 1997; Robinson, Loomes, & Jones-Lee, 2001; Sharma et al., 2002; Sung et al., 2003); 2) utility scores being greatly influenced by the choice of respondents (Ubel, Loewenstein & Jepson 2003); 3) important theoretical problems (Brousselle & Lachaine, 2009) presented by the standard gamble, a recommended instrument for calculating the utility score (CADTH, 2006); and 4) significant ethical dilemmas around discounting costs and effects at the same rate, whereas the choice of rate is arbitrary (Brousselle & Lachaine, 2009; Keeler & Cretin, 1983; Redelmeier, Heller, & Weinstein, 1994; Weinstein & Stason, 1977). No satisfying answers have yet been found to these long-standing and well-documented questions; nevertheless, these approaches and instruments have become increasingly accepted and used (Coast, 2004). To control for variability and uncertainty around assumptions underpinning the economic analysis or the use of debated instruments, sensitivity analysis is recommended (i.e., changing the values of some parameters to test the robustness of the original findings) (CADTH, 2006; Drummond, Sculpher, et al., 2005; NICE, 2008); however, doing so does not resolve these methodological difficulties. In fact, arbitrary values for some indices and standard procedures for conducting studies are recommended (CADTH, 2006; Drummond, Sculpher, et al., 2005; NICE, 2008), thus imposing a normative choice about what should constitute good economic evaluation practice without resolving the methodological debates.

Recent developments and complexity

Recent methodological developments include, among other things, the use of: decision-analytic modeling; sophisticated statistical techniques such as Markov models; calculations such as the ICER; the threshold approach to decision-making (a threshold being a critical value of one or more parameters above which a technology would be defined as not acceptable, Drummond, Sculpher, et al., 2005; NICE, 2008); and cost-effectiveness acceptability curves ("graphical representation of probability that intervention is cost-effective at various willingness-to-pay thresholds (or ceiling ratios), given the data available CADTH, 2006, p. A-12). These developments have not aimed at resolving the issues presented above, but rather have allowed for more sophisticated analyses that, in turn, opened up new debates on methodological complexity. According to Coast, "These methods are valuable, but by generating a pseudoscientific aura around economic evaluation, they camouflage critical weaknesses in current techniques" (Coast, 2004, p. 1233).

The current methodological developments increase analysis complexity at the expense of transparency (Weinstein, 2006) and may, in some cases, ultimately lead to inefficient resource allocation. For example, Birch and Gafni (2004, 2006a, 2006b) strongly criticize using a cost-effectiveness threshold approach, as done by NICE, and using the ICER approach for recommending health care interventions. More specifically, these authors express

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