The Reluctance to Use Cost-Effectiveness Analysis in Regulatory Decision-Making

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Despite the tremendous growth in the performance and publication of cost-effectiveness analyses (CEA) and other forms of economic evaluation in health and medicine, some policy makers, especially in the United States, have shied away from its use. Moreover, in some cases regulatory policy has discouraged its use. This paper explores reasons for this situation.

Cost-effectiveness analysis in health and medicine

The rise in the number of economic evaluations of health and medical interventions has been well-documented (Elixhauser et al. 1993; Elixhauser et al. 1998). One recent review of the literature found over 3,500 cost-benefit and cost-effectiveness analyses published from 1991 through 1996 (Elixhauser et al. 1998). An international database that maintains information on health economic evaluations contains over 11,000 entries (OHE-IFPMA 1998).

Cost-effectiveness analysis (CEA) has emerged as the dominant approach to economic evaluation in health and medicine (Office of Technology Assessment 1994; Gold, Siegel, Russell, and Weinstein

1996), though other forms of analyses such as cost-benefit analysis (CBA), cost-minimization analysis, and cost-consequences analysis have also been used.

The appeal of CEA is that it yields a ratio—costs per unit of health effect achieved—that is relatively straightforward to interpret and that allows for comparisons across a broad spectrum of interventions. Cost-effectiveness analyses show the relationship between the resources used (costs) and the health benefits achieved (effects) for an intervention compared to an alternative strategy. The cost per effect (C/E) ratio reflects the difference in the interventions' costs divided by the difference in their health effectiveness (Gold et al. 1996). If ratios are estimated in similar terms, they can be compared to illustrate the most efficient ways to maximize health benefits in the allocation of limited resources.

In contrast, CBA requires the monetary valuation of health benefits, which presents measurement difficulties and raises ethical issues (i.e., placing a dollar value on life). Other approaches also have limitations. Cost-consequence analyses (in which components of incremental costs and consequences of alternative interventions are computed and listed without any attempt to aggregate the results) may offer advantages in terms of their understandability but lack standards for methodological practices and do not produce results that can be easily compared across studies. The performance of cost-minimization analyses, which are used to compare the net costs of programs that achieve the same outcome, are less common because of the stringency of the requirement that competing programs yield similar effects.

Uses of cost-effectiveness analysis

In some cases, cost-effectiveness analysis has been used explicitly by policy makers. In Canada, for example, guidelines for performing economic evaluations of pharmaceuticals have been published (Menon, Schubert, and Torrance 1996; Baladi, Menon, and Otten 1998). The purpose of the guidelines is to "achieve sustainable pharmaceutical resource allocation, effective pricing policies, and equitable drug coverage" (Menon et al. 1996). The guidelines process is managed by the Canadian Coordinating Office for Health Technology Assessment (CCOHTA), a corporation created by federal, provincial, and territorial governments in Canada.

The guidelines stipulate that economic evaluation should not be part of the federal regulatory review process but rather should be used to demonstrate the value and cost-effectiveness of products being considered for reimbursement. Over the past few years, CCOHTA has assessed some four to six pharmaceuticals per month and has conducted evaluations in numerous areas, including cervical cancer screening and

the use of new medications for the management of major depression and acute migraine.

In the United States, some private managed-care plans have developed and used similar guidelines (Integrated Pharmaceutical Services 1997; Regence 1997). In general, however, policy makers have been reluctant to use cost-effectiveness analyses explicitly. For example, the Canadian federal Medicare program, which provides health coverage to elderly and disabled individuals, has been unable to enact a regulation that would add cost-effectiveness to the criteria used in coverage decisions for new medical technologies. Policy makers in the state of Oregon encountered difficulties when they attempted to follow the cost-effectiveness paradigm too closely for use in their Medicaid program, which provides health care to low-income individuals.

Barriers to using cost-effectiveness analysis

There are a number of barriers to the explicit use of cost-effectiveness analysis. Results from a handful of surveys conducted over the past few years shed some light on the issue (Lax and Moench 1995; Zellmer 1995; Drummond 1995; Luce and Brown 1995; Luce, Lyles, and Rentz 1996; Steiner et al. 1996a; Steiner et al. 1996b; Sloan, Whetten-Goldstein, and Wilson 1997). While the surveys differ in their scopes, methodologies, and sample sizes, several main conclusions emerge.

Many decision-makers feel ill-equipped to evaluate the information

One barrier to greater use of CEA is a feeling among decision makers that they do not possess adequate knowledge or training. Zellmer (1995), for example, reported that almost 40 percent of health-care managers said that they were ill-equipped to analyze and compare pharmacoeconomic claims critically. In another survey (Sloan et al. 1997), 15 percent of respondents listed lack of knowledge as a reason that cost-effectiveness analysis is not used more often. A similar percentage stated that a better explanation of methods was needed if CEAs were to be more useful to hospital managers.

Decision-makers remain skeptical of the information because they fear bias on the part of the study sponsors

The credibility and reliability of studies is also perceived as a problem. Zellmer found that fewer than 20 percent of respondents agreed with the statement "the comparative pharmacoeconomic claims made by drug manufacturers generally meet high standards for reliability." Moreover, only 51 percent of respondents agreed with the statement

"my managed care plan is in a position to put pressure on manufacturers to conduct scientifically rigorous pharmacoeconomic studies." In interviews with 43 managed-care providers, Lax and Moench (1995) found that the top concern expressed was "bias" followed by "freedom to control the study" and the "validity of the study." In a study of 446 medical professionals in the United Kingdom, Drummond (1995) reported that the greatest barrier stated was that "industry funded studies are not credible," reported by almost 60 percent of respondents. In the study by Sloan et al., over 20 percent of respondents said that a way to make CEA more useful to hospitals was to sponsor independent research. Almost 40 percent of respondents in the survey by Drummond et al. said that an external reviewer was needed to critically review studies for decision makers.

Decision-makers emphasize the need for more timely information

In a survey of 231 managers of private health plans, Steiner et al. (1996) found that the greatest reported barriers to decision makers were "no timely effectiveness data," expressed by 90 percent, followed by "no timely cost-effectiveness data (70 percent) and "no timely safety data" (60 percent). Among the barriers to use of cost-effectiveness analysis reported by Sloan et al. (1997) was the fact that studies were published too late. Almost 30 percent of these respondents replied that one way to make CEA more useful to hospitals was to make studies available sooner.

Decision-makers want targeted information that is more relevant to their own decisions

Sloan et al. (1997) reported that the two greatest barriers to the use of cost-effectiveness analysis among hospital pharmacists were that the analyses were not targeted at drugs of interest (34 percent) or that the analyses did not apply to hospitals (28 percent). When asked how cost-effectiveness analysis could be made more useful to hospitals, the most frequent response was that studies should be made "generalizable to the hospital setting." Drummond (1995) found that among the barriers reported by respondents were that "savings are anticipated, not real," that the Department of Health in the United Kingdom is only interested in cost containment, not cost-effectiveness, and that they "couldn't take the long-term view."

Cost-effectiveness remains a secondary consideration after clinical factors

It is also important to keep in mind that cost-effectiveness remains a secondary consideration after clinical factors. Luce and colleagues

(1996) interviewed 51 managers of managed care plans and asked about the usefulness of information on clinical effectiveness, safety, cost of treatment, and cost-effectiveness. Rated on a scale from 1 (most useful) to 6 (least useful), clinical effectiveness (1.6) was thought to be most useful followed by information on cost-effectiveness (2.6) safety (2.7), and cost of treatment (4.0). Luce et al. (1996) also found that respondents gave higher ratings to information from clinical trials as opposed to information from retrospective reviews and models. On a scale of 1 (excellent) to 4 (poor), clinical trials rated highest (1.8) followed by retrospective reviews (2.1) and models (2.6).

Regulatory barriers

A final barrier to the use of cost-effectiveness information is regulatory. In the United States, the Food and Drug Administration has traditionally concerned itself with matters of safety and effectiveness and not cost-effectiveness. But the Agency has long held authority to ensure that information disseminated by drug manufacturers is not inaccurate or misleading. The emergence of cost-effectiveness analysis has thus confronted the Agency with a new dilemma: how does it regulate promotional materials containing claims about a drug's cost-effectiveness?

In 1995, the FDA's Division of Drug Marketing and Communications (DDMAC) issued draft guidelines on the issue (FDA-DDMAC 1995). The guidelines stipulated that pharmacoeconomic studies would be required to produce "an adequate level of precision, scientific rigor, and validity (both internal and external) to support the resulting claims" (FDA-DDMAC 1995). All comparative claims would be required to provide "substantial evidence" typically demonstrated "by two adequate and well-controlled studies . . . Computer and mathematical models would be acceptable only when well-controlled trials could be performed; intermediate health outcomes and quality-of-life measurements can be employed only with evidence of the scientific association" (FDA-DDMAC 1995).

The draft guidelines were problematic because they were overly prescriptive, because they put too much stock in randomized controlled trials (RCTs) for economic endpoints, and because they gave short shrift to modeling exercises, which lie at the heart of cost-effectiveness analyses. The guidelines were also limited in that they did not show sufficient appreciation for the growing sophistication of purchasers—particular managed care plans—in using pharmacoeconomic information (Neumann, Zinner, and Paltiel 1996). In addition, cost-effectiveness should not be held to the same standard as safety and efficacy because the danger to consumers is an economic risk—paying too much for the benefits conferred—and not a health or safety risk.

Staff at the United States Federal Trade Commission (FTC) put forward many of these concerns, arguing that strict adherence to RCTs could result in the prohibition of truthful, non-deceptive claims of cost-effectiveness. They suggested a more flexible substantiation for economic claims based on "competent and reliable evidence" without an *a priori* specification for well-controlled trials (Neumann et al. 1996). "Competent and reliable evidence," they noted, might include epidemiologic or administrative claims, as long as adequate disclosure was provided (Neumann et al. 1996).

Legislation on the matter was addressed in the FDA Modernization Act of 1997. The provision states that "health care economic information provided to a formulary committee, or other similar entity . . . with respect to the selection of drugs for managed care or similar organizations . . . is based on competent and reliable evidence (US Congress 1997). While this legislation might offer some relief, it remains unclear how the "competent and reliable" evidence standard will be interpreted. Moreover, the FDA has yet to offer interpretive policy. A key issue in the debate is how well the FDA believes consumers are able to understand the information and separate "good" from "bad" information. In the meantime, pharmaceutical companies will likely remain reluctant to make claims about the cost-effectiveness of their products, despite a demand for such information on the part of health-care payers.

The road ahead

Considerations of cost will always play an important role in health-care decisions, whether they lurk in the shadows or are appraised openly. The real question is how explicit we are in using such information. Since cost-effectiveness analysis can help to illustrate how to improve health with society's limited resources, more efforts should be undertaken to make such information available.

To date, public-policy officials have shied away from using cost-effectiveness analysis as an explicit tool of policy making. Part of the problem is a lingering perception that the field lacks standards, making it difficult to compare cost-effectiveness ratios across studies. Some observers have also been troubled by the fact that many analyses are sponsored by drug companies with an interest in obtaining favorable results.

In recent years, progress has been made in addressing these concerns, including the publication of guidelines covering both methodological practices and the independence of researchers. To enhance the comparability of analyses, leaders in the field now suggest that cost-effective researchers undertake a "reference case" analysis, which involves a standard set of methodologic practices (Gold et al. 1996). New

guidelines suggest that analyses consider the societal perspective for the reference case and that they add other perspectives if they are important to the decision at hand.

The government can improve upon these efforts by sponsoring more research in the field, ensuring that this research adheres to the high standards recommended by leaders in the field, and allowing its dissemination to the public. The private sector can help by establishing mechanisms for independent, third-party review of cost-effectiveness claims. Editors of peer-reviewed journals would enhance these efforts by establishing more rigorous protocols for reviewing cost-effectiveness analyses prior to publication.

A more difficult hurdle for cost-effectiveness analysis is that its candid use raises the specter that health care is being "rationed." Here too, public-policy makers can help by leading efforts to educate the medical and health-policy communities about the usefulness of cost-effectiveness analysis in helping to maximize a nation's health tools under constrained health budgets.

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