Medical technology assessment, which traditionally has involved evaluating whether a technology was safe and effective, has been expanded in recent years to include consideration of cost-effectiveness. While most analysts and policymakers would agree that cost-effectiveness is, in principle, a worthy goal, actually using such a standard in coverage decisions is much more difficult. This Commentary focuses on the challenges involved for public payers and what we can realistically expect a cost-effectiveness criterion to accomplish. First, we briefly discuss the cost-effectiveness principle. Second, we address some of the key issues in assessing medical technologies. Third, we review how different public payers in the United States and overseas are addressing these issues. Finally, we discuss both the opportunities and limitations of cost-effectiveness analysis.

The Cost-Effectiveness Principle

Although formal methods for using cost-effectiveness analysis have been promoted to the medical community since at least the 1970s, costs have played a secondary role in technology assessment until recently. In the past few years more explicit considerations of costs have come to the fore. There has been an ongoing expansion of technology assessment’s focus from questions of “Is it safe?” and “Does it work?” to those involving the previously inviolate notion of “Is it worth it?” Medical journals now routinely publish cost-effectiveness analyses. Moreover, policymakers have begun trying to make such analyses an explicit part of the coverage process for new medical technology.

Cost-effectiveness analyses begin with the presumption that the objective of medical technology is to improve health. These analyses are in-

Peter Neumann, senior research director at Project HOPE’s Center for Health Affairs, is on leave working as a health economist on health reform at the Office of Management and Budget. Magnus Johannesson is an assistant professor of health economics at the Stockholm (Sweden) School of Economics and an adjunct assistant professor in the Department of Health Policy and Management, Harvard School of Public Health.
tended to show the relationship between resources used (costs) and the health benefits achieved (effects) for a given technology. The objective of such analyses is to illustrate how we can maximize the health benefits of a given amount of resources. While this goal may sound rather innocuous, operationalizing a cost-effectiveness standard is difficult. A host of theoretical and political issues await payers that attempt to use cost-effectiveness analyses explicitly in making coverage decisions.

**Issues In Cost-Effectiveness Analysis**

**Theoretical and methodological issues.** A critical issue in cost-effectiveness analysis involves the existing alternative to which a technology is compared. Presumably, a new technology will be compared with one that it likely will replace, but this is not always known at the time of the analysis. Take hypertension treatment as one example: A large number of different drugs are used, and it is not clear what the relevant comparison would be for a new drug.

Even when the competing alternative is known, judging whether a technology is cost-effective can be difficult. A technology may be cost-effective in some patient groups but not in others. Researchers have noted, for example, that the cost-effectiveness of hypertension treatment varies widely in different patient groups, depending on age, sex, initial blood pressure, and the presence of other risk factors. This problem may be possible to handle by good study design and the right indications for use. Another problem is that technologies constantly change and improve, making it difficult to rely on one-time assessments.

Another important issue involves how to define costs for purposes of analysis. While it is relatively straightforward to estimate the direct costs of using a technology (for example, the cost of equipment, supplies, physician time, and so forth) and the direct costs of competing alternatives, technologies usually also involve indirect costs and benefits (such as their impact on a person’s ability to work) as well as longer-term induced costs or savings, all of which are more difficult to estimate. Moreover, existing cost-effectiveness analyses differ widely in how they define costs.

An issue in any analysis is that costs and health benefits rarely occur at the same time. A technology may involve high initial costs, for example, but may yield health benefits (in terms of life years saved) far into the future. Typically, analyses are conducted in present-value terms, weighting future costs and benefits by a discount factor to make them comparable. But there are no standards regarding the length of patient follow-up to be considered. Moreover, analysts do not always agree on the precise discount rate to use or whether future health benefits such as life years should
be discounted, although these decisions can influence results. For example, studies of the cost-effectiveness of osteoporosis screening have illustrated that the discount rate used can greatly affect results.

Incorporating patient preferences into an analysis is one of the most challenging issues in cost-effectiveness analysis. Analysts have long argued that such preferences should be considered because a year of life with, say, a chronic disability, is valued by patients differently than a completely healthy year is valued. In recent years, researchers have substantially improved our ability to measure outcomes such as functional status, degree of disability, and emotional health. Many studies have attempted to consider quality-of-life or utility measures, but their use remains controversial. Critics argue, for example, that persons often possess ambivalent or imprecise preferences or that their attitudes and tastes change over time or depend on how a question is framed.

**Administrative and political issues.** Beyond the methodological challenges, there is the daunting task of selecting technologies to evaluate in the first place. This process generally involves three elements: health importance, economic importance, and an expectation that an assessment will make a difference. In 1992 Congress stipulated that the Agency for Health Care Policy and Research (AHCPR) would consider such factors in setting priorities for their own health care technology assessments. Actually quantifying the factors is more difficult, however, although some progress has been made. Charles Phelps and Stephen Parente, for example, have constructed an “index of expected gain” from technology assessment, which combines measures of resource use, variation in use rates across regions, and the rate at which the incremental value of a medical intervention changes as its rate of use changes. The index predicts that technology assessment should have a greater expected benefit when (1) large numbers of people are affected (health importance); (2) the per unit cost of the intervention is large (economic importance); and (3) the level of uncertainty is high and the marginal value of the technology falls rapidly as its use expands (assessment is likely to make a difference).

Another issue concerns the quality of the data. Data often are unavailable or incomplete. The source of the data is itself an important issue. Potentially, manufacturers could submit cost data to the government agency conducting the cost-effectiveness analysis, as pharmaceutical companies now submit clinical data to the Food and Drug Administration (FDA). This possibility underscores the need for the government to standardize reporting requirements and to ensure adequate verification and evaluation of submissions. Another thorny problem with some products and services involves what price to use in analyses and whether manufacturers and providers should be bound by the analysis price in marketing and
solving their products and services. Beyond methodological issues, the most formidable hurdle may be a political one. Adding an explicit cost-effectiveness criterion invites heightened public scrutiny. An example can be seen in the experience of the Health Care Financing Administration (HCFA), which proposed in 1989 to use an explicit cost-effectiveness criterion in approving medical technologies for Medicare. The regulation was withdrawn for further study in the election year of 1992 and has yet to be finalized.

Experience Of Public Payers

**United States: Medicare.** HCFA’s proposed regulation to add cost-effectiveness to technology assessment illustrates how a public payer in the United States may approach the issue of cost-effectiveness. In its proposed regulation, HCFA first defended its legal authority to use cost-effectiveness with a broad interpretation of a clause in the original Medicare statute that prohibited payment for expenses incurred for services “which were not reasonable and necessary for the diagnosis or treatment of illness or injury or to improve the functioning of a malformed body member.” HCFA added that it was also on firm policy ground because it believed that “considerations of cost are relevant in deciding whether to expand or continue coverage of technologies, particularly in the context of the current explosion of high-cost medical technologies.” HCFA qualified its stance, however, by explaining that “cost-effectiveness is one of several potential factors to be weighed in a given situation” and by proposing “to use a flexible definition of cost-effectiveness that encompasses a wide range of impacts.” HCFA further noted that it would not necessarily consider cost-effectiveness in every coverage decision and that it was “aware that cost-effectiveness analysis is a complex field that suffers from data limitations and the inability to quantify some costs.”

Lastly, HCFA proposed the analytic steps it would follow, noting that these steps are “well-accepted among economists.” The steps included (1) considering relevant alternative technologies to which current interventions will be compared; (2) identifying all relevant costs expected from the intervention; and (3) considering nonquantifiable factors. HCFA stated that it would study further how to collect and analyze primary and secondary data for cost-effectiveness.

**Australia: drug guidelines.** Since January 1993 Australia has required economic evaluations in applications for new drugs to be included in the national drug reimbursement scheme. For prescription drugs to be reimbursed in Australia, they must be included on the Pharmaceutical Benefits Scheme (PBS), operated by the federal government. If a drug is to be listed
on the PBS, an application must be made to the Pharmaceutical Benefits Advisory Committee (PBAC), which makes recommendations to the Australian government about listing new drugs.

The new requirement has its roots in a 1987 amendment to the National Health Act that required the PBAC to consider both costs and effectiveness in recommendations about listing new drugs in the PBS. After the legislative change, a group of experts was engaged to assist in the implementation of the new policy. This resulted in draft guidelines, issued by the federal government, for the preparation of submissions to the PBAC. The guidelines were revised once, and the inclusion of an economic evaluation in all submissions to the PBAC was made mandatory in January 1993. Economic evaluations are required only for new drugs. When the PBAC recommends a listing, it also passes on satisfactory economic evaluations to a separate pricing authority (the Pharmaceutical Benefits Pricing Authority, or the PBPA).

The Australian guidelines contain a number of important features. First, they state that the reimbursement decision and thus the economic evaluations should be based on a societal perspective—that is, taking into account all costs and benefits regardless of to whom they accrue. Second, they state that new drugs should be compared with the therapies that they are most likely to replace in practice. Third, they suggest that results of the economic evaluation should be presented both with and without indirect costs (production losses). Fourth, they note that there is inadequate experience to recommend utility measures in policy formulation, although they give cautious support to the continued development of such measures and suggest that the number of quality-adjusted life years (QALYs) gained should be used as the measure of effectiveness. Fifth, they encourage the use of intermediate effectiveness measures such as change in blood pressure for antihypertensive drugs, Finally, they recommend that the PBAC develop “yardsticks” to compare the cost-effectiveness of different drugs and to determine whether a cost-effectiveness ratio should be considered “high” or “low” for purposes of decision making.

**Canada: drug guidelines.** Economic evaluations are not yet required in Canada, although policymakers have been working to develop guidelines. In 1991 guidelines were issued in Ontario proposing economic evaluation requirements as part of submissions to the Drug Quality and Therapeutics Committee (DQTC). A revised version has since been issued for all of Canada (that is, all of the provincial drug programs).

In Canada each province has a drug benefit program that subsidizes the use of drugs by its residents. The drugs that are reimbursed are listed on different formularies in each province. In Ontario the minister of health decides which drugs should be included in the Ontario Drug Benefit For-
mulary, after advice from the DQTC, which considers effectiveness, safety, and cost in its recommendations.

As is true for the Australian guidelines, the Canadian guidelines adopt a societal perspective. Analysts also are encouraged, however, to conduct separate analyses showing results when only those costs funded by the provinces are considered. The guidelines state that new drugs should be compared with the cheapest alternative treatment for the same patients, but that comparisons with other treatment strategies also may be appropriate. The guidelines propose using QALYs to measure effectiveness, although they encourage experimentation with other approaches such as cost/benefit analysis where benefits are based on persons’ willingness to pay.

The Canadian guidelines include a system to assess the quality of the economic evaluations. A checklist of questions is provided against which the economic evaluations are to be assessed. These questions address the quality of the evidence and the independence between the investigators conducting the economic evaluations and the sponsoring drug company. The guidelines also recognize that if the cost per QALY is measured, it will be necessary to determine society’s willingness to pay per QALY gained in order to reach a recommendation based on the economic evaluation. Tentative thresholds for this judgment—that is, whether a cost-effectiveness ratio is considered “high” or “low”—were published in the initial version of the guidelines but were removed from revisions.

**Guideline and coverage comparisons.** From a methodological viewpoint, there are many similarities between the Australian and the Canadian guidelines, but also some important differences. The Canadian guidelines incorporate quality assessment of the economic evaluations, whereas the Australian guidelines are silent on this point, Also, the Australian guidelines encourage the use of intermediate effectiveness measures, while the Canadian guidelines do not.

From a policy perspective, the Australian and Canadian guidelines are similar. In both countries the registration, reimbursement, and pricing of drugs are separated; economic evaluations are intended as an aid to decisions about reimbursement. In Canada, however, prices are set before the decision about reimbursement, whereas in Australia the reimbursement decision precedes the pricing decision. Moreover, reimbursement decisions are made on the provincial level in Canada and on the national level in Australia.

**Europe: drug reimbursement.** According to a recent survey of health economists in Europe, economic evaluations are now used in a number of European countries as an input into decisions about drug reimbursement. An example involves reimbursement decisions for simvastatin and pravastatin in the Netherlands. Based on an economic evaluation, these drugs
were restricted to persons with high cholesterol levels who had one or more additional cardiovascular risk factors. However, formal requirements for economic evaluations as a basis for reimbursement decisions do not yet exist in any European country, although some attempts have been made in recent years to add standards for economic evaluations in Europe.

Discussion

Policymakers have long debated whether new medical technology is the culprit behind rising health care costs. Although they do not agree on the magnitude involved, experts generally concur that new technology is at least partly to blame; its contribution to rising health spending is usually put at between 10 percent and 40 percent. But this debate has been misguided.

First, technologies by themselves do not raise costs. The more meaningful question involves the health system in which technologies are used and whether the system encourages the adoption of important new technologies. Second, whatever the profile of the system, the problem is not whether costs are rising per se, but what is being achieved (in terms of health benefits) for the resources consumed. It is important to keep these points in mind when considering what a cost-effectiveness criterion can and cannot accomplish.

Ideally, a cost-effectiveness criterion can play an important role by guiding more sensibly the march of new technologies into the population. Implementing an economic criterion also will create an incentive for producers to develop cost-effective products in the first place. But ultimately, the underlying system and the incentives it embodies will largely determine the extent to which new technologies are developed, marketed, and used in clinical settings, regardless of whether an explicit cost-effectiveness provision exists in the coverage process. Moreover, a finding that a new technology is cost-effective will not assure use or reimbursement. How a technology is coded, classified, and priced by the system once a coverage decision is rendered will be critical factors.

It should be emphasized that cost-effectiveness analysis cannot resolve the question of whether society should pay for a new technology. It is only possible to compare cost-effectiveness ratios across technologies. To determine whether a technology should be covered, it will be necessary to determine how much society is willing to pay for the health benefits derived from the technology. Conducting cost-effectiveness analyses will not remove the need for difficult resource allocation decisions. But explicitly illuminating the trade-offs involved should help the process; thus, it represents a prudent step forward in public policy.
NOTES


3. We define technology broadly to encompass drugs, devices, and medical and surgical procedures.


20. Ibid., 707.
23. Federal Register 54, 4304.
24. Ibid., 4308-4309.
25. Ibid., 4309.
26. Ibid.
31. Ibid.
32. Ibid.
33. Ibid.