Information on Cost-Effectiveness: An Essential Product of a National Comparative Effectiveness Program

American College of Physicians*

The American College of Physicians recently highlighted the need to provide increased information comparing the effectiveness of health care interventions to ensure the rational and effective practice of medicine. Comparative effectiveness refers to the evaluation of the relative clinical effectiveness, safety, and cost of 2 or more medical services, drugs, devices, therapies, or procedures used to treat the same condition. The College further recommended the establishment of an adequately funded, trusted national entity that should prioritize, sponsor, or produce both comparative clinical and cost-effectiveness data.

This article addresses the need for the proposed entity to develop cost-effectiveness information. It examines the current reluctance to develop and use cost-effectiveness in the United States; it argues for the importance of this information for all health care stakeholders; and it makes specific recommendations regarding how this information can best be made available and used for the good of the public and our patients.


Comparative effectiveness is the evaluation of the relative (clinical) effectiveness, safety, and cost of 2 or more medical services, drugs, devices, therapies, or procedures used to treat the same condition. After reviewing comparative effectiveness efforts in this country and internationally, the American College of Physicians (1) concluded that the United States expends insufficient funds to develop comparative effectiveness data; that no coordination or prioritization of current efforts exists in either the public or private sector to produce comparative effectiveness information; and that the absence of readily available comparative effectiveness information interferes with the ability of physicians and their patients to make effective, informed treatment choices that meet the unique needs and preferences of the patient and facilitate the ability of payers to optimize the value of their health care expenditures.

The College called for an adequately funded, trusted national entity to prioritize, sponsor, and produce comparative effectiveness and cost-effectiveness information. Others have recently made similar recommendations (2–7). The American College of Physicians recommended that this entity be protected from undue government and private sector influence, provide transparent proceedings and reports, include extensive stakeholder involvement, and implement processes to ensure the general distribution of comprehensible findings. Furthermore, the College recommended that the entity produce both comparative clinical and cost data, in the strong belief that both factors are critical to making health care resource decisions for all stakeholders.

This article explores the availability and use of information that compares clinical outcomes and cost. It argues that the national entity should develop and use clinical information that compares the health care outcomes of plans of care, along with their costs, to ensure the most effective and efficient use of limited health care resources.

Cost Information in Comparative Effectiveness Analyses

Investigators use various terms and analytic techniques (such as cost-identification analysis, cost–consequence analysis, and cost–benefit analysis) when cost factors are a significant aspect in the comparison of the effectiveness of different medical interventions (8). The most frequently used technique, cost-effectiveness analysis (CEA), compares the incremental or marginal economic cost per unit of health care gained among different interventions for the same condition without attempting to monetize the health care gain. A cost-effectiveness analysis provides a single ratio, the incremental cost-effectiveness ratio, that reflects the difference in the costs of interventions (in U.S. dollars) divided by the difference in their health effectiveness or clinical outcomes.

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\text{Incremental Cost-Effectiveness Ratio} = \frac{\text{Cost}_{\text{intervention A}} - \text{Cost}_{\text{intervention B}}}{\text{Health Outcome}_{\text{intervention A}} - \text{Health Outcome}_{\text{intervention B}}}
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Cost-Effectiveness Information and a National Comparative Effectiveness Program

Position Paper

Health outcomes may be defined in various ways, such as life-years gained, cases of disease prevented, improved functional status, or quality of life. The measure recommended by the Panel on Cost-Effectiveness in Health and Medicine (9) is health-related quality-adjusted life-years (QALYs). A QALY assigns a weight ranging from 0 (death) to 1 (perfect health), corresponding to the patient’s quality of life, for each period. Quality-adjusted life-years provide a common currency to assess the benefits or burdens that patients experience in terms of quality and quantity of life. Because this metric is a ratio, it can grow large either because its numerator (the incremental cost of the intervention) is large or, perhaps more important, because its denominator (the incremental effectiveness of the intervention) is small. Because the costs of an intervention have a finite limit, the largest incremental cost-effectiveness ratios (and hence the most inefficient uses of limited resources) occur when the more expensive intervention provides little or no health benefit.

The Reluctance of Policymakers to Use Cost-Effectiveness

The literature supports the development and use of evidence of relative clinical effectiveness; however, support for using cost-effectiveness data is more limited. This was most recently reflected in the Medicare Payment Advisory Commission’s (MedPAC) June 2007 Report to Congress (4), which highlighted the need for increased comparative effectiveness information and recommended the development of a national entity to compare the clinical effectiveness of treatment approaches. Although the production of cost-effectiveness information was not ruled out, the MedPAC Commissioners showed substantial ambivalence about including such data during the public meeting where they reviewed this recommendation (10).

Through its rulemaking process, Medicare has twice attempted to endorse the use of cost-effectiveness data in coverage decisions. On both occasions, strong, broadly based concern was expressed, including the fear that such use would be the forerunner to rationing of care (8), and Medicare abandoned its efforts. That policy directly affects traditional Medicare (Parts A and B) and private plan Medicare (Part C), which must provide at least the basic traditional benefits and must abide by all Medicare national coverage decisions that expand coverage (11). The new Medicare drug coverage benefit (Part D) provides greater flexibility for private drug plans to use costs in formulary decisions.

Traditional state Medicaid programs are restricted in applying cost or cost-effectiveness data to their formularies (12). Medicaid programs must cover all drugs approved by the U.S. Food and Drug Administration from every manufacturer that signs an agreement with the Secretary of Health and Human Services to pay rebates to the states for the drugs purchased; however, preferred drug lists can be established if specific requirements are met. States do have the flexibility to employ cost and cost-effectiveness evidence in deciding whether to require prior authorization or other utilization management procedures. The Drug Effectiveness Review Project (www.ohsu.edu/drugeffectiveness) provides such information. State Medicaid programs are directly influenced by cost factors. State budgets go through cycles of surplus and deficit. During periods of deficit, coverage under Medicaid and other state-funded health programs is often reduced, typically by not covering specific populations or benefits that are not mandated under the specific program. Oregon attempted to use cost-effectiveness to prioritize Medicaid benefits in the early 1990s, but subsequently minimized the influence of costs on the prioritizing process after substantial stakeholder criticism (8). Only Washington has a program that formally assesses safety, efficacy, and cost-effectiveness to inform these decisions (13).

Commercial health plans and purchasers are also reluctant to use formal CEA, even though 90% use costs in some form when evaluating new interventions (14). For example, the health insurance industry uses budgetary impact analyses, in which new technologies are evaluated in terms of their projected effect on cost per member per month, often without relating cost to patient outcomes (Weinstein MC. Personal communication).

In a survey of private U.S. health plans, only 40% indicated that they use formal CEAs (14). In another survey, only 51% of private payers used CEAs or cost–benefit analyses (15). Similarly, the Blue Cross Blue Shield Association’s national Technology Evaluation Center generally excludes cost-effectiveness considerations (16). When cost-effectiveness data are used within the commercial sector, such use is typically limited to pharmaceutical coverage and pricing decisions.

As summarized below, several reasons have been proposed for the observed reluctance to develop and use cost-effectiveness.

Fears about Access to Services

Patients and their advocates are concerned that use of any cost data, including formal CEAs, will inappropriately limit access, be used primarily for cost containment, and be a substantial step toward rationing of care (8, 17, 18). The reaction of Medicare’s beneficiaries to the agency’s attempts to allow limited consideration of cost-effectiveness is a good example. These fears are particularly strong in the United States, where the belief that the richest nation in the world should not consider cost in decisions about access to tests and treatment seems to persist (18). This opposition often focuses on cost-effectiveness data rather than cost data alone. Many insurers already use information about cost because they must manage finite budgets. Of note, the strongest opposition seems to come from such constituencies as elderly persons and patients with certain
chronic illnesses, for whom the incremental benefits of expensive interventions are typically small.

Opponents of the use of cost data neglect to recognize the unsustainable growth in health care expenditures. Medicare’s trustees have expressed concerns over the past several years regarding the financial stability of the Medicare system (19). Furthermore, health care premium growth continues to far outpace growth in the economy and workers’ earnings, making health care increasingly unaffordable to both employers and employees (20).

Rationing is already occurring. More than 47 million people in the United States are uninsured for health care, primarily because they cannot afford the insurance premiums (21, 22). This lack of insurance decreases access to needed health care and produces substantially poorer medical outcomes (23).

Health care resources are limited, even in this rich country, and cost already plays and will continue to play a role in health care expenditure decisions. Making valid and reliable cost-effectiveness data from a trusted source available to all stakeholders would provide a needed tool for both the general population and traditional health care purchasers (for example, government programs and employers). Such a tool could improve value and result in a better, more socially equitable means of controlling health care costs than our current system, which limits access for some of our most vulnerable, needy citizens and uses cost information in a nontransparent manner that does not often consider effectiveness. Cost-based decisions seem to occur largely for interventions that are expensive (when the incremental ratio has a large numerator), rather than for relatively ineffective interventions (when the incremental ratio has a small denominator).

Recent research literature indicates a broadly based, renewed interest in increasing the availability of cost-effectiveness information. With appropriate safeguards, the majority of the general public believes that use of cost-effectiveness data when making policy is a reasonable approach to controlling the escalating rise in health care costs and copayments (18). This interest in cost and cost-effectiveness should continue to increase as the employee’s share of health care costs increases and high-deductible insurance products coupled with health saving accounts become more prevalent. Most physicians now agree that they should consider cost-effectiveness when making clinical decisions (18). Both the Blue Cross Blue Shield Association (24) and America’s Health Insurance Plans (25) have recently called for efforts to increase the production of cost-effectiveness data from a trusted source. The National Business Group on Health provides its members with cost-effectiveness data within their guidelines for covered services (26). In addition, many other countries that perform higher on health care benchmarks than the United States (27, 28), including Canada, Great Britain, Germany, France, Sweden, and Australia (2, 3, 29), use cost-effectiveness in their health care coverage and pricing decisions.

Great Britain’s National Institute for Health and Clinical Excellence (NICE) program is the most recognized international entity that produces both comparative clinical and cost-effectiveness information (30).

Soundness of Analytic Methodology and Trust in the Data Currently Being Produced

Traditional CEAs leave a great deal of discretion to the researcher, and differences in analytic design decisions can lead to substantially different results. For example, a recent MedPAC-commissioned review of studies on the cost-effectiveness of colorectal screening compared with no screening indicated incremental cost-effectiveness ratios ranging from $1400 to more than $42 000 per life-year gained (13). The same reviewers found that incremental cost-effectiveness ratios for implantable cardioverter defibrillators compared with pharmaceutical treatment ranged from $18 000 to $569 000 per life-year gained (13). Trusting a methodology that produces such varying results for the same treatment may be difficult.

The study design decisions left to the discretion of researchers conducting CEAs include perspective of the analysis (for example, society, insurers, or purchaser); sources of the data (for example, taken directly from clinical trial results, from studies reported in the literature and integrated by a decision model, or from claims data); discount rate used to adjust for changes in the value of future costs and benefits; costs to include in the analysis; time over which to consider benefits and costs; selection of interventions compared; selection of populations considered; form of outcome measurement used (for example, QALYs, number of life-years gained, gains in functional capabilities, cases diagnosed, or deaths avoided); and how to express the uncertainty of clinical events and costs.

In 1993, the Public Health Service convened a group of nongovernmental scientists and scholars with expertise in cost-effectiveness to recommend improvements in the quality and comparability of reported CEAs. The Panel on Cost-Effectiveness and Health viewed CEAs as an “aid to decision making, not a complete procedure for making resource allocation decisions,” called for increased transparency in study reporting, and recommended a “reference case” reflecting a standard set of methodological practices that an analyst should follow in conducting cost-effectiveness studies (9). Although variations continue to occur, a review of more recently published CEAs indicates improved comparability among the studies (31). The MedPAC has recently concluded that this continued variability should be further addressed before Medicare can routinely use CEAs (13).

In addition, industry-funded CEAs may be biased—they are more likely than non–industry-sponsored research to report cost-effectiveness data that favors adoption in routine practice (8). Neumann (8) reported that “virtually all surveys of health decision makers” reveal concerns about the presence of bias attributable to industry. The New En-
gland Journal of Medicine instituted a policy limiting its consideration of industry-sponsored CEAs to those that were funded through a grant to a nonprofit organization, that provided assurances of author independence, and that included information on all assumptions made and all data used (32). Other journals have taken similar, although more limited, steps.

Cost-effectiveness analyses can and should report all of their assumptions and computations. In a properly conducted and reported analysis, this transparency could lead to more rational, consistent, and accountable choices than the behind-closed-door decisions of some current coverage policies.

Inhibiting Technical Innovation in Health Care

Might the use of CEAs in coverage decision processes slow down or inhibit innovation (8, 17, 33)? This concern is particularly directed toward Medicare because of its purchasing power. Denial of coverage would have a large adverse financial effect on a company and might make it more averse to the risk of investing in potential technical advances. Neumann (8) countered that innovation depends on multiple factors, including incentives offered by payers, society’s overall willingness to spend money on health care, the available supply of venture capital funds to support investment, and the rigidity with which cost-effectiveness thresholds are applied in policymaking decisions. He contends that the use of cost-effectiveness data does not necessarily inhibit innovation but instead may actually stimulate the development of more cost-effective interventions.

Potential for Increase in Litigation Initiated by Beneficiaries

Tunis (17) highlighted the difficulty Medicare would have in justifying coverage denials to their beneficiaries that were based even in part on cost factors. Health plans may be reluctant to use CEAs because such use may lead to an increase in litigation (8, 16). Most current health plan contracts, although they emphasize the use of clinical effectiveness evidence within the coverage decision process, make little or no reference to the use of cost-effectiveness as part of the coverage decision process. In addition, beneficiaries often react negatively to coverage denials, and some sue. Little litigation has challenged the use of CEAs, and health plans have successfully defended challenges to other resource-containment initiatives, establishing important legal precedents (8).

A Case for the Use of Cost-Effectiveness

The MedPAC recently highlighted the potential for use of CEAs to complement current clinical effectiveness considerations in Medicare’s coverage and pricing processes to obtain increased value for its medical expenditures (29). The Commission further outlined potential cost-effectiveness activities under Medicare, including collecting cost-effectiveness information as part of the coverage decision process, sponsoring cost-effectiveness studies, providing results of CEAs to beneficiaries and sponsors to help assess the relative value of different treatments, and using that information to set priorities among various agency initiatives (such as when to use pay-for-performance to encourage behavior change, which populations to focus on for disease management interventions, and which conditions to target with health care screening).

Cost-effectiveness information is a necessary complement to comparative clinical effectiveness information for all health care stakeholders. This information will help patients and their physicians make treatment decisions that better reflect the needs and preferences of the patient and support the profession’s commitment to a just distribution of finite resources (34). It will provide relevant information for health care payers and plans to help ensure value from their expenditures. It will also serve as a stimulus for medical innovation and technological advances that take the relative value of new equipment or procedures into account. Cost information is already being used to make decisions about health care coverage, rate setting, tiering, and utilization management decisions, but not always in a transparent, explicit manner. The more these decisions can be informed by both cost and clinical effectiveness, combined in an explicit, transparent manner—something offered by CEA—the higher the likelihood of obtaining true value and equity within the health care system.

Many of the concerns currently inhibiting the production and use of cost-effectiveness information can be adequately addressed, are not supported by factual information, and should not continue to inhibit the availability of such information. Furthermore, several of these concerns apply equally to evidence of clinical effectiveness but have not inhibited the production of such evidence.

All relevant stakeholders should be good stewards of our health care resources so that those resources can be used to facilitate the availability of effective care (34). Cost-effectiveness information can provide an important tool toward this goal.

Recommendations

Recommendation 1: A national comparative effectiveness entity should be established and charged with systematically developing both comparative clinical and cost-effectiveness evidence for competing clinical management strategies.

Given the general sensitivity toward the use of cost-effectiveness information among some stakeholders, the limited understanding among the general public, specific concerns regarding the methodology, and the potential for inappropriately restricting access to necessary health care, we further recommend the following:

Recommendation 2: The national comparative effectiveness entity should convene a panel of stakeholders and experts...
in CEA and charge the panel with updating and expanding on the recommendations of the 1993 Panel on Cost-Effectiveness and Health (9) and developing procedures to ensure that the proposed entity produces high-quality cost-effectiveness information.

Recommendation 2A: The panel should develop recommendations and model procedures to be used by stakeholders as they consider cost-effectiveness information in coverage, purchasing, and pricing decisions. These recommendations should recognize that CEA is only one tool to be used in coverage and pricing decisions; it cannot be the sole basis for making resource allocation decisions.

Recommendation 2B: The panel should consider how physicians should use cost-effectiveness in the context of the physician–patient relationship to reflect the need for patient care to be patient-centered, considering the individual's characteristics and preferences, and should take into account the opinions of the treating physician as the patient’s advocate. However, it must also recognize the limited nature of health care resources available to society (the medical commons) (35).

Recommendation 2C: The panel should develop recommendations for educating both the general public and the medical profession and for promoting discussion on the use of comparative clinical and cost-effectiveness information to meet the needs of the individual and to help ensure the equitable distribution of finite health care resources throughout society.

A panel of medical and pharmacy directors of health plans recently offered a strategy for introducing cost-effectiveness information more explicitly into healthy policy and coverage decisions (36). Given the unsustainable growth in health care expenditures occurring in this country, we recommend the following:

Recommendation 3: All health care payers, including Medicare, other government programs, private sector entities and the individual health care consumer, should consider both comparative clinical effectiveness and cost-effectiveness information explicitly in their evaluation of clinical interventions.

Recommendation 4: Cost should never be used as the sole criterion for evaluating a clinical intervention. Any consideration of cost must be explicit and transparent and must be accompanied by the explicit, transparent consideration of the comparative effectiveness of the intervention.

**Conclusion**

Given the as-yet uncontrolled explosion of health care costs, the de facto rationing produced by having 47 million uninsured patients denied access to health care, and the limited resources of our society, the time has come for patients, physicians, insurers, and health care policymakers to explicitly and transparently factor the comparative effectiveness, comparative cost, and cost-effectiveness of both new and existing health care interventions into their decisions. The United States should establish a trusted, independent, adequately funded national entity to develop and disseminate evidence on comparative costs, comparative effectiveness, and cost-effectiveness in health care and to educate the public about the urgency of modifying our cultural bias toward ignoring the cost of health care. The economic effects of interventions should be used in decision making, but only if their use is transparent and their effect on health outcomes are also considered.

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