



# Research Insights

## ■ Incorporating Costs into Comparative Effectiveness Research

### Summary

Comparative-effectiveness research attempts to establish the relative health benefits of different drugs, medical devices, diagnostic and surgical procedures, diagnostic tests, and medical services as a tool to improve health care outcomes and quality. Cost-effectiveness analysis, usually expressed as the cost of a medical technology per quality-adjusted life year (QALY) achieved, is a formal economic tool for comparing the relative value of medical technologies. Perspectives about the appropriateness and methods of incorporating costs into comparative effectiveness research differ greatly.

Proponents of examining costs alongside health outcomes point to the fact that costs are already a part of health care discussions including decisions about coverage and payment for health care services and budgetary deliberations about public sources of insurance. They argue in favor of making these considerations systematic and transparent.

Among the arguments against incorporating costs into comparative effectiveness analysis are public discomfort and political challenges to using cost-effectiveness for decision-

making, inherent biases in cost-effectiveness analysis against new and less well-proven technologies, and difficulties in measuring costs and health benefits. They also argue that reducing uncertainty in health care through clinical effectiveness research is a more valuable use of scarce resources than is cost analysis.

Alternatives to formal cost-effectiveness analysis for incorporating cost considerations into comparative effectiveness research include analyzing higher cost services first and requiring higher standards of evidence for high cost services. Pay-for-performance programs and other innovations in payment policy are yet other strategies to promote the use of higher value services.

For comparative effectiveness research that does explicitly incorporate costs, there are a variety of “best practices” and prior experiences to draw upon. In addition to several states that use cost-effectiveness analysis to guide their public insurance programs, almost all industrialized countries’ health insurance schemes use estimates of the relative value of health care services to make coverage and payment decisions.

### Genesis of This Brief: AcademyHealth’s 2009 National Health Policy Conference

AcademyHealth convened a panel of experts with differing perspectives on the incorporation of costs into comparative-effectiveness analysis during its annual National Health Policy Conference (NHPC) in Washington, D.C. in February 2009. Steven Pearson, M.D., director of the Institute for Clinical and Economic Review (ICER) at the Massachusetts General Hospital and Harvard Medical School; Kathy Buto, currently with Johnson & Johnson and formerly with the Centers for Medicare and Medicaid Services (CMS) and the Congressional Budget Office; Gerard Anderson, Ph.D., Bloomberg School of Public Health at Johns Hopkins University; and Mark Gibson, Center for Evidence-Based Policy at the Oregon Health and Science University (OHSU), participated in the panel. Michael Chernew, Ph.D., from the Harvard Medical School moderated the discussion.

## Introduction

Policymakers, stakeholders, and experts who seek to use comparative-effectiveness research as a mechanism to improve health care quality and efficiency must consider what role analysis of the costs and value of health care services should play in this effort. Promoting value in health care — i.e. maximizing the health benefit achieved for each dollar spent — is one strategy used to slow the growth in health care spending. Policymakers in other countries and in some U.S. states already take costs or value into account when deciding which health care services insurance should cover or how much to pay for them. However, perspectives about the appropriateness and methods of incorporating costs into comparative-effectiveness research differ greatly.

## What are Comparative-Effectiveness Research and Cost-Effectiveness Analysis?

Comparative-effectiveness research attempts to establish the relative value of different drugs, medical devices, diagnostic and surgical procedures, diagnostic tests, and medical services. It is intended as a tool to improve health care outcomes and quality.<sup>1</sup> At a minimum, such research compares the clinical effectiveness of one health care technology or service with another. It provides information about which of the two technologies produces better outcomes for a particular patient or group of patients. The concept of “value” brings health care costs together with clinical effectiveness; distinguishing “high value” services from “low value” services requires the measurement of costs.

One formal economic tool for making such comparisons among services is cost-effectiveness analysis. Researchers usually express the relative cost-effectiveness of a service in terms of its cost in achieving a unit of health benefit, usually an extra year of life or quality-adjusted year of life (QAYL). Measuring costs and estimating QAYLs (or other measures of health benefits) can present technical difficulties, be carried out using different methodologies, and generate varying opinions about the appropriateness or usefulness of cost-effectiveness as a tool to inform clinical care or policy.

## Why Incorporate Costs Into Comparative-Effectiveness Research?

Proponents of examining costs or cost-effectiveness alongside health outcomes in comparative effectiveness research make several arguments in favor of their point-of-view:

→ As cost containment occupies a more prominent place on the health policy stage, information that distinguishes between high and low value health care services helps direct spending to its most efficient uses. All else being equal, when two treatments are equally effective, one would want to use the less expensive option.

- Cost considerations are already implicitly a part of many clinical, coverage, and payment decisions. Incorporating costs into comparative effectiveness research assures that these considerations are transparent to patients, providers and payers.
- Unless costs and value are made part of the public dialogue about health care services now, there will be no societal support for considering costs and value in the future when financial or budgetary pressures will force difficult choices about how much should be spent on health care and for what services.
- State governments are already facing budgetary pressures that force hard choices about eligibility and benefits for Medicaid and other state programs. Cost-effectiveness analysis, if well done, has the potential to improve quality of care for individual patients at the same time it helps spend scarce public dollars efficiently.
- Unless costs and value are made a part of comparative effectiveness research, health care purchasers and insurers will continue to face difficulties in negotiating prices for services and technologies that reflect their incremental benefits.
- Unless considerations of cost and value are made transparent, patients, providers, and the public may believe that health plan coverage decisions and efforts to guide clinical decision-making have been made only in an effort to save money.

## Recent U.S. Initiatives to Promote Comparative Effectiveness Research

Section 1013 of the Medicare Modernization Act (PL 108-173) mandated that the U.S. Agency for Healthcare Research and Quality (AHRQ) conduct and support research with a focus on outcomes, comparative clinical effectiveness, and appropriateness of pharmaceuticals, devices, and health care services. The agency implemented this mandate through its Effective Health Care program ([www.effectivehealthcare.ahrq.gov](http://www.effectivehealthcare.ahrq.gov)). In the 110th Congress, Senator Max Baucus (D-MT) introduced the Comparative Effectiveness Research Act of 2008 (S. 3408), a bill that is expected to be reintroduced in the 111th Congress and would establish a federal trust fund and an independent entity to conduct such research. More recently, the American Recovery and Reinvestment Act of 2009 (PL 111-5) provided an additional \$300 million to AHRQ, \$400 million to the National Institutes of Health, and \$400 million to the Office of the Secretary of the Department of Health and Human Services for comparative effectiveness research.

→ Without consideration of costs and value, manufacturers in the health care marketplace have an incentive to pursue innovations that carry a high price without regard to their marginal health benefits.

Observers who have concerns about making cost analyses a part of comparative effectiveness research offer several arguments in favor of their position:

→ Reducing uncertainty in the provision of patient care is a higher societal priority than is distinguishing services in terms of their cost-effectiveness. Hence, clinical effectiveness research that improves quality of care is a better use of scarce resources than are cost analyses.

→ The public is more comfortable knowing that physicians are making clinical decisions for individual patients as opposed to having a large government or private entity making decisions for an entire population of patients.

→ The United States has a diverse health care payment system in which costs vary across different payers, which complicates the measurement of the costs of specific health care services. If costs vary by payer, then so too will cost-effectiveness estimates vary. In addition, different payers may have different thresholds of cost-effectiveness in making coverage decisions.

→ Using cost-effectiveness analysis as a tool for making clinical and policy decisions is politically infeasible because the public sees it only as a means to *limit* coverage.

→ When incremental cost-effectiveness calculations are used to guide decisions about the coverage of new technologies or services, the analysis inherently favors treatments already covered. The burden of proof falls on the new technology.

→ Similarly, there is a presumption against less well-proven innovations. A lack of evidence about the clinical or cost-effectiveness of a service is inherently equated with evidence of a lack of benefit.

→ Cost-effectiveness calculations are based on population averages and can underestimate the value of services for individual patients.

→ Cost-effectiveness methods are imperfect and can vary in how they measure costs or QALYs or other metrics of value.

→ Economists have not proven that new technology is a major driver health care cost increases. Analyses of health care spending trends *assume* that technological change explains cost increases not attributable to other known causes. According to this critique, cost containment efforts would be more effectively focused on the known drivers of increased spending: (1) geographic variations in care, (2) chronic illness, and (3) the fee-for-service (FFS) payment system, which always rewards doing more.

→ Medicare, the country's largest payer, could use cost-effectiveness analyses in setting reimbursement rates, but it is not well positioned to use them in determining coverage policy because of the political expectation that Medicare will continue to offer the same benefits to all enrollees (even if only a small percentage of enrollees might benefit from a given technology).

→ Analyses that used Medicare cost data would not yield real cost-effectiveness calculations since the program only collects proxy measurements of hospital costs.

### Alternatives to Cost-Effectiveness Analysis

Even without conducting formal cost-effectiveness analyses, it is still possible to incorporate costs into comparative-effectiveness research. One option is to prioritize services to be analyzed for their clinical effectiveness so that higher cost services are given a higher priority. Another possibility is to require higher standards of evidence of clinical effectiveness for higher cost services when making coverage decisions. A third option is to use cost, but not cost-effectiveness in making decisions based on comparative effectiveness. For instance, if there is no good evidence that one drug is clinically more effective than another, decision makers could consider them comparable and choose the cheaper alternative.

In addition, Medicare could take steps on its own to encourage the use of services based on value. It could offer conditional coverage for particular services, limiting their use to circumstances, settings, or providers for which research has established that they are clinically effective. Second, Medicare could base payments to providers on their meeting quality or efficiency standards established by clinical effectiveness research. Medicare already uses this approach, referred to as “pay-for-performance” (P4P) or “value-based purchasing” to some extent for hospitals and on a voluntary basis for physician services. Expanding P4P would help diffuse “best practices” among Medicare providers. And finally, Medicare could develop new

policies that deal directly with Medicare's known cost-drivers, especially chronic disease and FFS payment.

### How Should Cost-Effectiveness Be Incorporated Into Comparative-Effectiveness Research?

Experts have suggested several “best practices” for policymakers and researchers who decide to make costs or cost-effectiveness a component of their comparative-effectiveness research.<sup>2</sup>

- The methods employed for incorporating costs into comparative effectiveness research should depend on how the information will be used. The first question should be “What decision are we trying to make?” and then, “What information best supports that decision?”
- Because costs vary by provider and location, researchers should focus on collecting cost data that reflect the population that will potentially receive the services under study.
- Determining costs often requires laying out complex clinical pathways that depend on intermediate outcomes. Hence, estimates of the overall costs associated with a service require knowing the costs associated with each pathway and averaging them to reflect the probabilities of each occurring. Specifying complex clinical pathways can be a difficult and resource-intensive part of comparative effectiveness research.
- The public is less likely to oppose cost and cost-effectiveness analysis when they see it as only a tool to help *inform* treatment options than when they believe it is being used to *make* coverage decisions.
- Researchers should do both cost- and clinical-effectiveness analysis in a transparent way to assure public trust in the quality of the research.
- If costs are to be taken into account in comparative effectiveness research, policymakers need to decide organizationally who should do it and with what governance structure. One option would be for individual payers, including Medicare, to commission cost and cost-effectiveness research. The downside of this option is that patients, providers, and the public may distrust the analysis since payers have a stake in its outcome. A second option would be to fund cost analyses as part of the overall comparative-effectiveness effort, but have the analyses undertaken by an allied, separate organization. A third alternative is to have a single organization carry out both the clinical and cost analyses as part of a jointly funded effort. Although this may be the most efficient way to carry out

the work, it could lead to distrust of the clinical-effectiveness results because they are mixed with cost analysis that patients, providers and the public may not trust.

- Although clinical effectiveness is often examined without incorporating cost into the research, the costs of two services should never be compared without simultaneous consideration of their relative clinical benefits.

### Other Countries' Experiences

Other countries consider costs in comparative-effectiveness research; a recent study of 10 industrialized countries drew five general conclusions.<sup>3</sup> First, the study found that all of the countries' comparative-effectiveness research programs explicitly include costs in their analyses. A few of these programs added cost considerations to their methods sometime after their founding. Second, each country's stated purpose for considering cost was to promote value, not to achieve savings. Third, most countries' research adopts the perspective of a payer, although a few say they do their analysis from the perspective of society as a whole. Fourth, most countries' comparative effectiveness research involves syntheses of published literature that can combine studies that are done from differing perspectives. For example, studies that rely on claims data have a payer perspective, but randomized controlled trials may have payer, patient, or societal perspective. And fifth, methods used to measure costs vary from country to country and usually reflect the purpose for which the research is intended. In some countries, comparative-effectiveness research is used to inform or determine coverage policy; in others, it is used to inform the amount paid for a particular drug, device or procedure.

The experiences of three countries in particular illustrate some of the diversity of approaches to handling costs. In Australia, comparative-effectiveness research is used to determine the clinical equivalence of drugs. If a new drug is equivalent to an existing drug and results in lower costs, health insurance will cover it. The national agency responsible for this research has existed since 1911, but it has only considered costs since 1990.

In most of the United Kingdom (England and Wales), the National Institute for Clinical Excellence (NICE), established in 1997, analyzes the clinical- and cost-effectiveness of drugs, devices, treatments, and public health measures. Their research is the basis for coverage decisions for particular technologies under the UK's National Health Service. For most services, there is a \$30,000 to \$40,000 per QALY threshold, beyond which, NHS does not provide coverage. Some conditions, like cancer and rare diseases, have higher thresholds. These cost-effectiveness

## Assessing Comparative Effectiveness and Value: ICER's Integrated Evidence Rating System™

The Institute for Clinical and Economic Review (ICER) based at the Massachusetts General Hospital has developed a systematic approach to integrating the results of comparative effectiveness and “comparative value,” which ICER defines as a “judgment largely based on the incremental cost-effectiveness of the technology being appraised.”<sup>4</sup>

In assessing comparative effectiveness, ICER combines judgment about the magnitude of net benefits of the technology – i.e. the overall balance between benefits and harms – with the level of confidence in the evidence supporting the assessment. In comparing the effectiveness of one technology against another, ICER classifies it into one of six categories:

- A** Superior (High confidence of a moderate-large net health benefit)
- B** Incremental (High confidence of a small net health benefit)
- C** Comparable (High confidence of a comparable net health benefit)
- D** Inferior (High confidence of an inferior net health benefit)
- U/P** Unproven with Potential (High confidence of at least comparable health benefit and limited confidence suggesting a small or moderate-large net health benefit)
- I** Insufficient (The evidence does not provide high confidence that the technology gives patients at least as much net benefit as does its comparator.)

In evaluating comparative value, ICER assesses the differences in utilization, system impact, and cost-effectiveness of alternative treatment pathways. In head-to-head comparisons, ICER classifies technologies into one of three categories reflecting their relative value: high, reasonable or comparable, and low. There are no strict thresholds linked solely to estimates of cost-effectiveness; rather, ICER analyzes cost-effectiveness and uses it as a component of an overall judgment of comparative value.

To facilitate dialogue about value, and to render its assessment more able to support innovative patient decision aids, clinical guidelines, and health plan policies concerning coverage and reimbursement, ICER combines comparative effectiveness and comparative value in an “Integrated Evidence Rating”™ that places a technology into one of the cells on the following table:

Comparative Clinical Effectiveness	Comparative Value		
	High (a)	Reasonable/Comparable (b)	Low (c)
Superior (A)	Aa	Ab	Ac
Incremental (B)	Ba	Bb	Bc
Comparable (C)	Ca	Cb	Cc
Inferior (D)	Da	Db	Dc
Unproven with Potential (U/P)	Ua	Ub	Uc
Insufficient (I)	I	I	I

By arraying particular medical technologies in this manner, ICER highlights how their clinical benefits “come at varying relative values based on their cost and their impact on the outcomes of care and the health care system.”<sup>5</sup>

thresholds are not specified in law; rather, they are derived from the body of past NHS coverage decisions. NICE estimates that its analyses of services cost, on average, about \$250,000.

The German Institute for Quality and Efficiency in Health Care (IQWiG), established in 2004, conducts clinical- and cost-effectiveness studies as a condition for insurance reimbursement in that country. A joint committee of representatives from Germany's different sickness funds makes coverage decisions, and each sickness fund sets a ceiling ("reference") reimbursement price for clinically equivalent treatments using IQWiG's cost-effectiveness estimates. These reference prices vary by disease and sickness fund.

### The State Perspective

States face competing pressures as they set eligibility and benefits for Medicaid and other insurance programs within their budgets. On the one hand, patient advocates and industry want maximum coverage of services, and on the other, taxpayers want to limit expenditures. Because this dilemma forces states to deal with the cost of health services as they make health spending decisions, some states have begun to use cost-effectiveness analysis to guide them. The studies that states use are primarily funded by the vendors who manufacture the technologies under consideration. In addition, experts have observed that the evidence of clinical effectiveness included in these cost-effectiveness models is often of dubious quality, and the studies often employ complicated, quantitative modeling techniques when simple comparisons of technologies would be sufficient to inform policy. And finally, policymakers and the public are often resistant to studies that employ complicated metrics like QALYs since their meaning and the methods used to calculate them are not necessarily intuitive.

Despite these limitations, experience suggests that cost-effectiveness analyses can help policymakers pragmatically set priorities among health care services. For example, OHSU's Center for Evidence-Based Policy, the Drug Effectiveness Review Project (DERP) and the Medicaid Evidence-Based Decisions Project (MED), provide reviews of clinical- and cost-effectiveness health care technologies for states and other payers. ICER also conducts reviews of particular clinical services and technologies for the State of Washington. As part of this work, ICER most recently analyzed virtual colonoscopy and coronary computed tomography angiography.

### About the Author

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### Endnotes

- 1 U.S. Medicare Payment Advisory Commission, *Report to the Congress: Reforming the Delivery System*. 2008. Washington, DC: US Government Printing Office, p. 107.
- 2 Like the other points summarized in this issue brief, participants in AcademyHealth's panel on "Incorporating Costs into Comparative-effectiveness Research" suggested these "best practices." AcademyHealth National Health Policy Conference. (February 3, 2009). Washington, DC.
- 3 The countries are Australia, Brazil, Canada, England and Wales, France, Germany, the Russian Federation, South Korea, Sweden, and Turkey.
- 4 Institute for Clinical and Economic Review. Methodology: ICER Integrated Evidence Rating™. Monograph, 2008. [www.icer-review.org/index.php?option=com\\_content&task=blogsection&id=13&Itemid=42](http://www.icer-review.org/index.php?option=com_content&task=blogsection&id=13&Itemid=42). Accessed on February 27, 2009.
- 5 Institute for Clinical and Economic Review, *op. cit*