

Why Don't Americans Use Cost-Effectiveness Analysis?

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Cost-effectiveness analysis (CEA) offers decision makers a structured, rational approach with which to improve the return on resources expended. But decades after its widespread promotion to the medical community, policy makers in the United States remain reluctant to use the approach formally. Indeed, the resistance to economic evidence in the United States in an era of evidence-based medicine in healthcare is perhaps the most notable development of all. This paper examines the resistance to CEA in the United States and explores ways in which to advance the field.

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Cost-effectiveness analysis (CEA) logically should occupy an important place in the health policy-making toolkit. The technique promises to increase the return on society's investment in healthcare. It provides a tool to inform decisions in an explicit, quantitative, and systematic manner. Not only do medical journals routinely publish CEAs,¹⁻³ but many countries have incorporated CEA into their technology assessment and reimbursement procedures.⁴

In theory, CEA should be of use to managed care organizations, insurers, health departments, and state and federal policy makers.⁵ But despite the widespread promotion of CEA to the medical community, US policy makers remain reluctant to use the technique.

After repeated attempts to incorporate cost effectiveness as a criterion for covering new medical technologies, Medicare abandoned the pursuit in the face of political opposition.⁶

In another example, Oregon Medicaid initially sought to rank services based on cost effectiveness. But the plan was opposed on ethical, legal, and political grounds and was implemented only after officials removed the offending cost-effectiveness provisions.⁷ In the years since, no other state Medicaid program has attempted to implement Oregon-style priority setting.

Why have managed-care plans not embraced CEA? Given their turbulent finances and attempts to coordinate healthcare under fixed budgets, one might expect them to be eager consumers. Instead, studies suggest otherwise.^{8,9} Health plans have employed evermore aggressive and sophisticated processes for managing care

from incentive-based formularies to utilization review policies. But few, if any, providers use cost effectiveness as a formal policy tool.

It is also difficult to find explicit use of CEA in other corners of US health policy. Clinical guidelines incorporate economic analyses infrequently, even when high-quality evaluations are available.¹⁰ Health insurance contracts use the term "medically necessary," but not "cost effective."¹¹ In general, researchers find little relationship between the cost effectiveness of lifesaving programs and their implementation.¹²

In this paper, I examine resistance to CEA in the United States and offer advice to policy makers. Full disclosure: I receive grant funding to review and occasionally conduct CEAs. However, my intention in this paper is not to advocate CEA, so much as to understand its place in the health policy field and its prospects for the future.

UNDERSTANDING RESISTANCE TO CEA

One explanation for resistance to CEA holds that American physicians fundamentally do not understand—and have not been taught to think deeply—about resource constraints and tradeoffs.^{13,14}

A second theory points to lack of a trust in the methods, rather than a lack of understanding per se. Researchers have long observed that many published CEAs do not adhere to recommended protocols for conducting and reporting,^{3,15} which may contribute to a sense that the methodology is not ready for prime time.^{16,17}

A related problem is a perceived lack of relevance. Observers point to the disconnect between the abstract, societal-perspective of recommended CEAs, and the short-term horizons of actual decision makers.^{18,19}

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Critics argue that CEA studies neglect the budget impact of the service in question.²⁰ Promised savings are perceived as illusory.

A more cynical explanation questions not the methods of CEA but the motives of investigators and/or sponsors. On the one hand, CEA is seen as a smokescreen for cost-cutting efforts. Physicians view CEA as an accounting or cost-management tool used by managers to improve the bottom line.^{14,18,21} On the other hand, CEA is perceived as a tool with which those with a financial gain at stake, particularly the drug companies who increasingly sponsor studies, can advocate to *increase* health expenditures.¹⁶ Yet another explanation points to legal and regulatory barriers as key factors.¹¹

But each of these explanations falls short. Lack of understanding or mistrust of methods and motives undoubtedly plays a role. Still, why have policy makers in the United States not funded or conducted their own CEAs, or tailored them to their own needs as they have in other countries?

Legal and regulatory factors also fail as a full explanation. Nothing in federal statute explicitly bars Medicare from using CEA. Similarly, nothing prevents private health insurers from writing contracts that specify covered services as those that are deemed “medically necessary and cost effective.” Plans may fear lawsuits if they use CEA openly. But there are also plausible reasons to believe that health plans could withstand these challenges, as they have withstood challenges to other cost-containment initiatives.¹¹

The best explanation is that, at its roots, resistance to CEA in the United States is grounded, not in methodological or legal barriers, but in Americans’ deep-seated distaste of limits and of the corporate or government officials who impose them.

At some level, people do not believe that resources really are limited, or they recoil from the explicit nature of the cost-effectiveness exercise itself—that it forces them to think consciously about stark tradeoffs between money and health that they would rather leave at a subconscious private level.¹³ Physicians believe using CEA violates clinicians’ advocacy duties, destroying the trust necessary for good doctor–patient relationships.¹⁴

Moreover, CEAs may not capture public preferences for allocation of limited resources. As Daniels and Sabin point out, while intended as an empirically based, transparent methodology relying on evidence, CEA is not value neutral.²² It carries with it morally controversial and, to some, unacceptable assumptions. Empirical evidence suggests that patients and their physicians desire equity and fairness in a general sense, rather than efficiency per se.²³⁻²⁵ They want to give priority to patients with life-threatening treatments or to children or the

disabled, even when treatments are not cost effective.^{14,26,27} Application of CEA is seen as violating the special moral importance of health or as breaching citizens’ inherent rights to healthcare.^{21,28}

Objections to CEA may be particularly acute in the United States for several reasons. For one, survey data suggest that compared to their counterparts in Europe or Canada, Americans are more concerned about access to the most advanced medical technologies.²⁹ For another, CEA may be seen as especially objectionable in the United States’ private and often for-profit healthcare system. Consumers and providers may believe that these economically driven, sometimes publicly traded organizations are not motivated or inspired by a desire to maximize the value of healthcare services to members of their plans. They may believe that the overall health system reflects unwise and inefficient use of resources, and they may therefore be unwilling to forego potentially beneficial care simply because of cost. People have demonized managed-care plans for trying to limit patients’ choice of provider and treatment based on economic considerations; they may view CEA as a formal, explicit methodology to achieve the same end.

Other countries’ acceptance of CEA confirms that the United States’ failure to use CEA is driven more by the country’s own cultural, political, and institutional conditions than by the technique’s inherent methodological shortcomings.

DOES ANYONE USE CEA? EXAMPLES FROM THE FIELD

But how to reconcile the resistance toward CEA with the rapid growth of analyses in mainstream American medical journals? Why would analysts continue publishing studies if the information was assiduously ignored?

CEA may actually enjoy considerable influence in the United States, not as an explicit instrument for prioritizing health services, but as a subtle influence in policy discourse. Over the years CEA has challenged prevailing wisdom and brought clarity to healthcare debates—underscoring, for example, that prevention programs usually do not produce cost savings³⁰ and that sometimes high-tech, cost-increasing interventions can ultimately provide very good value for the money.³¹

Most health plans and formulary managers have an interest in CEAs; and almost all large medical-products companies now have sophisticated health economic units, reflecting a widely held view that customers now consider this information when making purchasing decisions. Health plans have begun adopting the Academy of

Managed Care Pharmacy's (AMCP's) new evidence-based formulary guidelines that call for drug manufacturers to submit dossiers of clinical and economic evidence about their products to support the listing of new pharmaceuticals.³²

CEA may also be influencing selected clinical guidelines increasingly. For example, CEAs have shown statin drugs to be relatively cost effective as secondary prevention in persons with existing heart disease, but considerably less cost effective as primary prevention.³³ While formal recommendations did not follow these CEAs strictly, the economic analyses were cited in the guidelines and likely play a role in targeting therapy.^{5,34}

More importantly, several national initiatives now consider CEA formally, including the recent recommendations of the third US Preventive Services Task Force (USPSTF) and the Guide to Community Preventive Services.^{35,36}

IMAGINING A FUTURE FOR CEA

Has CEA had any influence in the 25 years since its introduction to the American medical community?³⁷ Of course. By its sheer existence, CEA changes the nature of conversations about the impact of investments in healthcare. It forces and focuses discussions about the value of health and medical services within a clear theoretical framework. It generates a more careful consideration of available evidence and sheds light on how to target resources to particular clinical practices or subgroups of patients.^{38,39} The thousands of articles in peer-reviewed literature over the years attest to its currency among researchers and thought leaders.

At the same time it has fallen short of expectations. Visions of using CEA as a formal tool for prioritizing health services never materialized. But excessive pessimism about the prospects of CEA would be misplaced too.

US policy makers have gravitated toward a policy of "cost effectiveness once removed." The rules of engagement allow use of CEA but only at a safe distance. Rationing is permitted under the radar. Physicians preserve the fiction that everything is being done for their patients, even as they play a supporting role in apportioning scarce societal resources. At the same time, patients safeguard their trust in their doctors. Payers maintain legal protection.

Ubel observed recently that "cost-effectiveness analysis has had, at best, a troubled youth... but it will give way to a successful adulthood."¹⁴ He went on to predict that administrators, policy makers, and others will become more adept at using CEA.

This may yet happen. The United States may witness a broad cultural shift in the way policy makers think about priority setting.⁴⁰ Practicing physicians may begin to embrace CEA openly as a tool for bedside rationing. Payers may begin appealing more directly to evidence from CEAs when writing contracts or targeting services for coverage. The public may become more acceptant of the technique, especially if alternative approaches to rationing, such as bureaucratic obstacles and long queues, are seen as even more objectionable.

But these scenarios seem unlikely. If anything, recent history points to a backlash against the imposition of limits. The public's appetite for managed care has long been eroding.⁴¹ Unlike our neighbors to the north or across the ocean, the United States seems destined to keep CEA at bay, defying the trend as we have resisted the metric system.

The more probable future is one in which cost-effectiveness information is produced in abundance but rarely used in explicit fashion to cover or deny services. Instead, employers and health insurers will likely continue to impose limits in other ways.

ADVICE FOR THE US POLICY MAKER
AND POLITICIAN

More and better training about CEA in schools of medicine, public health, pharmacy, and nursing would help the field and improve the quality of CEAs. So too would renewed attempts to improve the peer-review process.^{17,42}

A strong, independent organization to conduct or evaluate CEAs would also be beneficial. Public organizations, such as the USPSTF, have made some progress. Government agencies, such as the Agency for Health Care Research and Quality and the National Institutes of Health sponsor selected CEAs.⁴³ However, a clearly articulated policy for coordinating CEA research across agencies has never existed.

A stronger role for the FDA is possible, with the agency given authority to examine the cost effectiveness of prescription drugs before approval. But changing the FDA's approval authority would be a mistake and would embroil the agency in debates about value that are better left to the marketplace. Greater responsibility for the Centers for Medicare and Medicaid Services (CMS) seems natural, but experience has shown the enormous difficulties the agency would face.⁴⁴

A new agency within the US Department of Health and Human Services could be established, but political forces would conspire against it. A better idea is to create a quasi-public entity like the Institute of Medicine to judge

the cost effectiveness of new therapies, though it too would be hard-pressed to weather the political storms.

In the end, the best hope may be a decentralized reform like the emerging Academy of Managed Care Pharmacy formulary guidelines. Such reform could permit explicit considerations of value without the fallout of centralized government assessments.

US policy makers and politicians can also learn several lessons from the past.

CEA Should Not Be Used Rigidly

Leaders in the field have always warned against using CEA mechanically, but it took Oregon's misadventures to drive home the lesson. Oregon was viewed as being in the vanguard of health policy making, but the rest of the country never followed.⁴⁵ Expectations for CEA should be modest. In countries that have implemented CEA, subsequent decisions have been consistent with economic efficiency. The Australian reimbursement authorities have been unlikely to recommend a drug if the cost-effectiveness ratio exceeded AU \$76 000 per life-year saved and unlikely to reject it if less than \$42 000 per life-year saved.⁴⁶ An upper threshold of about £30 000 per quality-adjusted life-year seems to have emerged at NICE.⁴⁷ But exceptions have also been made, as in the case of riluzole for amyotrophic lateral sclerosis and beta interferon for multiple sclerosis.⁴⁸

CEA Will Not Save Money

CEA should never be conceptualized or promoted as a cost-containment tool but rather as a technique to obtain better value.⁵ Paradoxically, using CEA tends to increase health spending because it reveals more under- than overtreatment.³⁹ This truth will likely present an additional challenge for those advocating the methodology, but transparency on the matter will help manage expectations.

It Matters How You Say It

Research shows that physicians understand that resources are limited, but they are not willing to admit to rationing.¹⁴ Similarly, health plan managers deny that they ration care but admit that their budgets are constrained.¹⁸ These responses are instructive. It suggests that the term *cost effectiveness* is part of the problem. Insisting on an "eat your broccoli" approach—that Americans must simply come to grips with the need to ration—is unlikely to work.

Incentives First

Debates about the use of CEA cannot be divorced from debates about the underlying health system and the incentives the systems embody (eg, formularies

with tiered copayments that create incentives for patients to seek lower cost drugs). Value-for-money arguments may be more acceptable if used in a decentralized fashion by competing plans rather than centrally by CMS. But reconfiguring the incentives that face providers and patients is critical.

Think Broadly Across Sectors

A final message involves the importance of thinking expansively about applications of cost-effectiveness analysis. Cost effectiveness analysis should not focus simply on health and medical interventions but more broadly on interventions to reduce environmental, motor vehicle, and occupational risks as they too affect health outcomes.

Anticipating the Medicare Drug Benefit

To Medicare, CEA has been an elephant in the living room, officially ignored despite its obvious importance. A decade of failed attempts to integrate CEA has revealed the strength of reluctance in the United States to openly confront resource constraints. If Medicare officials—and politicians—learned anything from the ordeal, it is the political folly of trying to ration honestly. Despite rising Medicare expenditures and the impending retirement of the baby boomers, prospects for the adoption of CEA remain dim. Physicians are already angered and frustrated by CMS. Politicians will not risk antagonizing elderly voters.

At the same time, Medicare cannot avoid difficult decisions about costly new technology. What's a \$400 billion-agency to do? Most likely, it will stumble along with one hand tied behind its back, evading the tough choices by delegating authority, cutting payment rates, and handling expensive new technology through coding and payment policy, all the while reassuring beneficiaries that they will maintain access to important advances.

Recent high-profile national Medicare coverage decisions on implantable cardioverter defibrillators and lung volume reduction surgery are instructive. Despite vows that it does not use CEA, Medicare in both instances sought to limit its budget exposure by selecting populations for coverage, consistent with the notion that Medicare appeals to economic factors, if not explicit CEA, in making decisions.

All of this anticipates the newly enacted Medicare drug benefit that will take effect in 2006. Many questions remain about how private plans will make formulary decisions about new drugs. A key question is whether they will use CEA explicitly. History suggests that it will be difficult for them to do so. Tellingly, new legislation gives the Agency for Health Care Research and Quality new funds for cost-effectiveness research

but explicitly forbids CMS from using the information to withhold coverage of new drugs.

CONCLUSIONS

Various factors explain the resistance to CEA in the United States, including a lack of understanding about the conceptual approach, a mistrust of methods and motives, and regulatory and legal barriers. But the loudest message of all pertains to Americans' distaste of limits.

CEA's proponents have always assumed that increased spending and the public's appetite for medical technology will eventually force us to recognize limits more directly and that inevitably we will embrace CEA as the best solution to our dilemma. But Americans' reluctance to acknowledge limits—and their resistance to CEA—has proven remarkably durable. In many ways, CEA has emerged as a technical success but a political failure.

There are a few encouraging signs. Where use of the technique has emerged, however, it has done so not as the Oregon plan's architects envisioned but in more nuanced fashion. The most plausible scenario for the future is one in which coverage decisions reflect an untidy combination of evidence- and politics-based medicine and in which CEA plays a behind the scenes but not explicit role.

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