

Economic Decision Modeling in Health Care

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From the Editor

- Ethics in Economic Modeling in Health Care 599
Elina E. Pliakos

Case and Commentary

- How Should Willingness-to-Pay Values of Quality-Adjusted Life-Years Be Updated and According to Whom? 601
Paul T. Menzel, PhD
- How Should Economic Value Be Considered in Treatment Decisions for Individual Patients? 607
Hadley Stevens Smith, PhD, MPSA

Medical Education

- How Should Economic Evaluation Be Used to Measure Value and Set Priorities in Health Care? 613
Sahan Jayawardana, MSc and Elias Mossialos, MD, PhD

In the Literature

- "Perspectives" in Health Technology Assessment 619
Anthony J. Culyer, DEcon

State of the Art and Science

- How Economic Decision Modeling Can Facilitate Health Equity 624
Jeffrey S. Hoch, PhD, Logan Trenaman, PhD, Shannon M. Hearney, MPH, and Carolyn S. Dewa, PhD, MPH

Policy Forum

- How Should Economic Analyses Inform Nosocomial Infection Control? 631
Eleftherios Mylonakis, MD and Panayiotis D. Ziakas, MD, MSc, PhD

How Does Cost-Effectiveness Analysis Inform Health Care Decisions? David D. Kim, PhD and Anirban Basu, PhD	639
How to Motivate Equity in Health Decision Modeling Robert I. Field, JD, MPH, PhD	648
Art of Medicine	
American Value Kelsey Coolahan	653
How Valuable? Dahlia Fischer	656
An Animated Portrait of Inaccessibly High-Cost Care Taylor Colette Moon, MFA	658
Ironic Currency Kashvi Gupta, MBBS, MPH, Kehaan J. Saraiya, and Saurabh Jha, MD, MS	660
Personal Narrative	
Set Down the Stethoscope Michael Westerhaus, MD, MA	662
Podcast	
Economic Decision Modeling and Reducing Waste in Health Care: An Interview With Elina E. Pliakos and Dr Ankur Pandya	



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FROM THE EDITOR

Ethics in Economic Modeling in Health Care

Elina E. Pliakos

It has been estimated that approximately 25% of annual health care spending in the United States is unnecessary and wasteful.¹ This amounts to about \$760 billion to \$935 billion that could be saved annually and which, if saved, could help curb increasing US health care expenditures.¹ Various strategies have been proposed to decrease unnecessary spending, such as focusing on preventive care, eliminating unnecessary tests and procedures, and controlling costs of prescription drugs.² Health care system volatility and the ease with which vital resources are depleted in crises make it necessary to find reliable ways to allocate limited health care resources to maximize overall population health benefits while minimizing risk and harm. Resource allocation decisions about which interventions to invest in are fraught with complexity and uncertainty. Therefore, decision analytic models are often used to synthesize evidence from multiple sources and help inform decisions that must be made while navigating such complexity.³

Economic decision models aim to quantify clinical and economic benefits and harms associated with interventions to help policymakers and organizational leaders forecast prospective costs and manage likely trade-offs. In 1977, Weinstein and Stason suggested that resource allocation decisions should be made and **priorities set** based on indices of costs relative to anticipated benefits.⁴ Although computational abilities have improved since 1977 and advancements in medicine⁵ have led to development of guidelines about how to conduct health economic analyses,^{6,7} little attention has been given to ethical and social dimensions of using economic decision models and analyses in health care. This theme issue aims to fill this gap and considers how economic modeling can motivate good decision making about improving health systems performance, clinical practice, and patients' health care experiences.

Variations among standard care, evidence-based care, and value-based care can make it hard to decide which economic decision modeling guidance to follow when designing and implementing models and interpreting results generated by those models. Model structures, data sources, and assumptions, for example, influence the validity of what clinicians and organizational leaders can learn from them and are, therefore, ethically, socially, and culturally relevant. This theme issue explores this set of themes in detail and considers how transparency in modeling can help **motivate equity, cost-effectiveness**, good resource stewardship, and **value**. My hope is that this theme issue will illuminate key concepts at the intersection of economic modeling and health care

and stimulate discussion, so that we can offer all patients high-quality care in economically sustainable ways.

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CASE AND COMMENTARY: PEER-REVIEWED ARTICLE

How Should Willingness-to-Pay Values of Quality-Adjusted Life-Years Be Updated and According to Whom?

Paul T. Menzel, PhD

Abstract

Before updating any willingness-to-pay (WTP) per quality-adjusted life-year (QALY) threshold, a few points must be recognized. Ethical justification for using WTP thresholds and QALYs lies in incorporating the preferences of those whose treatment could be affected by resulting resource allocations. For WTP thresholds, such justification depends on the sufficiency of a match between a group—members of an insurance pool from which health care payments and services are drawn—and those whose health care is potentially affected. For QALYs, that justification depends on eliciting the right persons' preferences to inform quality-adjustment ratings; on balance it should be from those who have the conditions being rated. Because the value of simply being alive is not adequately accounted for, how life extension and quality improvement are combined in constructing the QALY is its most significant shortcoming as a measure. Although updating WTP thresholds might be better than not updating them, this manuscript suggests why drawing on a less fundamentally flawed concept than the conventional QALY is more important.

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Case

A willingness-to-pay (WTP) threshold, according to the World Health Organization (WHO), is a value used to represent “an estimate of what a consumer of health care might be prepared to pay for the health benefit” and is often based on a country’s per capita gross domestic product.¹ In the United States, a WTP threshold of \$50 000 to \$100 000 is still referenced and used today by public and private policymakers, insurers, and researchers, for example, despite having been established in 1982.²

One health care organization’s executive, Dr CXO, has suggested, “Given how insurers and other third-party payers in the US health care system rely on population-based WTP

thresholds to guide decisions, we should probably be leery of using estimates that are so old.”

Dr CXO continued, “If a patient or that patient’s physician, for example, asked me why we’ve been using decades-old value estimates to determine, say, what a patient’s additional quality-adjusted life-year (QALY) might be worth, I don’t think I’d be able to come up with a very convincing answer.”

Dr CXO then assembled a long-term task force charged with updating the WTPs the organization uses, particularly as they relate to QALYs. How the WTPs should be updated is, however, unclear to task force members as they begin to deliberate.

Commentary

To engage in the ethical debate about the current threshold value for a WTP/QALY ratio, or the willingness to pay for a QALY gained, and the importance of updating it, a clear understanding of the essential functions and nature of both WTP and QALY is required. Their ethical relevance lies in their reflecting the preferences of those whose health is directly affected by the **allocation of health care resources** that a given threshold guides. Both the “preference” and “those affected” aspects are important. The value of health care, to be sure, is not only the value of the care to those whose health is at stake, but its value to them is primary. Health care, after all, is primarily for its recipients, so their preferences must be prioritized.

What WTP and QALY Represent

A WTP/QALY threshold expresses preferences about the relative value of health care compared to things other than health care for which the same financial resources might be used. Providing an upper limit on what should be spent on any treatment or program is the threshold’s primary use. A secondary use emerges when the upper monetary limit of a QALY’s value is empirically derived for many services and treatments. Then the resulting values of cost/QALY ratios of various services and treatments can be compared and used to set priorities within health care.

The QALY itself represents trade-off preferences for 2 different kinds of health benefits. It is constructed to combine both life extension and quality-of-life improvement in a common unit of health benefit value measured on a 0 to 1.0 scale. To do that, people’s trade-off preferences between quality-of-life improvement and life extension must be elicited, typically by time trade-off (TTO) and standard gamble (SG) questions.³ TTO questions ask what portion of an anticipated remaining life with a given imperfect health-related quality of life—paraplegia, for example—one would be willing to sacrifice to regain full health. SG questions ask what chance of death a person is willing to take to regain full health. Essentially these are the right questions. How else would one get the subjective preference utility value of 2 different sorts of things into a common scale except by trade-off preferences? Such a health state valuation—the quality adjustment of the value of a year of life in different conditions—forms the empirical core of QALYs.

Whose WTP?

WTPs. Typically, most health care expense is paid through insurance, either private (individual or employer sponsored) or public (Medicare or Medicaid), or by direct public provision funded by the tax-paying public, such as the British National Health Service (NHS) or the US Veterans Health Administration (VHA). All are collective arrangements. The contributions of subscribers, employers, or taxpayers are pooled and then either

paid out to providers or used to fund a direct provision entity like the VHA or NHS. The individual connections between those who originally contribute the resources and those who receive the care do not need to be tracked. The relevant WTP preferences are therefore those of all funding contributors who might receive health care through the pool. The WTP threshold should therefore be the aggregate preference of the pool.

QALYs. In the denominator of the threshold (the QALY), whose preferences to use in determining how much quality-adjustment is appropriate for a year of life in a given condition gets complicated. Actual patients, particularly those with chronic illness and disability, rate their quality of life more highly than do “hypothetical patients,” who are only imagining themselves with the conditions they are rating.^{4,5,6,7} Adaptation and the more direct knowledge of those who actually have the conditions probably explain most of the difference.

The central argument for using hypothetical patient ratings is that health state valuation needs to incorporate everyone in the insurance pool. Everyone has a potential stake in the ratings, since anyone can sooner or later end up with one or more of the conditions being rated. Hypothetical patients should, of course, imagine as best and knowledgeably as they can what life for them would be like in the condition they are rating.

The case for using the ratings of actual patients, however, is stronger. Health state valuation is an attempt to get at the relative values of real conditions that patients will experience. Patients are the ones who actually do experience those conditions. Since it is their health and life in those conditions that is the real health and life at stake, if we want to get the preference utility of the real thing (why should we not?), the **primary data** need to come from actual patients. To be sure, many further factors complicate the choice of whom to ask, but they do not change the essential argument.^{6,7,8}

Deeper Problems

Deeper problems lie not in whose preferences to elicit but in the QALY's essential nature as a common unit of health benefit expressing trade-off preferences between quality of life and life itself. For treatments and services that reduce pain and suffering and improve quality of life, health state valuations from actual patients may work reasonably well in discerning the value that health-related quality-of-life changes have for them. The more difficult problem comes with the value of life itself—added or lost years.

The value of life itself—being alive at all for a given time, not the quality of life during that time—is life's value compared to not being alive—that is, compared to death. With death, however, everything of experiential value to the person is lost. Compared to death, then, any life short of the most difficult and despairing conditions that would make life not worth living can and often will assume enormous value. Something, when it's all one can get, is worth a very great deal compared to nothing. This alone will tend to equalize the subjective value to each individual of life extension in various conditions.

With one further element, the phenomenon of adaptation, we can see how compelling the claim is that for different persons across most health states, life itself has equal value. We already know that health state valuations by persons with a condition like paraplegia are higher than the ratings that people only imagining themselves with paraplegia give.⁹ The latter might be willing to trade 20% of their life expectancy to gain a cure, for example, while the former are willing to trade only 5% (a 0.80 rating compared to 0.95). The preference trade-off disparity is even greater for some with

disabilities, who are unwilling to trade any of their life expectancy to gain a cure.¹⁰ Such “no traders” insist that even with disability, life compared to death has full value (1.0).

Such “hedonic adaptation”¹¹ is also referred to as the “relativity of happiness,”^{11,12} and the structure underneath it as the “psychological immune system.”¹³ As important as it is, adaptation’s empirical limits should be recognized. People do not adapt much to what they see as temporary afflictions; for example, the adaptation effect is strong only for those with chronic conditions regarded as likely permanent.¹⁴

At the same time, these same persons with chronic conditions insist that quality improvement has value, too. The two are different dimensions. In answering TTO questions by being willing to sacrifice 1 of 20 years to gain a cure, for example, they are rating their quality of life at 0.95. They do see value in health status improvement. Yet a moment’s reflection reveals that life itself has as much value for them as it has for those who can survive with full health-related quality of life. In saying she would be willing to sacrifice 1 year of 20 if she could regain full limb function for the rest of her life, a person has not said that her life itself (that is, her life compared to death) has any less value *for her* than the life itself of the person without paraplegia has *for that person*. Even people without paraplegia who think about this, *when they really do see themselves in the shoes of the person with paraplegia*, can readily understand how that person can still value life as much as they themselves do.

But if the equal value of life is intractable, so is the value of quality improvement. The essential structure of the QALY, which combines the value of both quality improvement and life extension in a common unit of value measured on a 0 to 1.0 scale, seems not to represent the real value to people with imperfect health-related quality of life of both their life extension and their quality-of-life improvement. Calculations using the QALY, created by trade-off preferences between a shorter life with full health and a longer life with imperfect health, yield the conclusion that since the value of quality improvement from a cure is 0.05 for a person with paraplegia in the example above (1.0 minus 0.95), then the value of a year of life extension for the person with paraplegia must be 0.95, and thus the priority for saving that person’s life drops compared to the value of saving the life of a person in otherwise full health.^{8,15} If we pay careful attention to the real values of both quality improvement and life extension, however, priority for saving the life of the person with paraplegia should not be one bit less than the person without it.

Updating the Cost/QALY Threshold

If the QALY has these internal contradictions, then it’s right to question whether and when the value of the WTP/QALY ratio should be used. The QALY is the wrong thing to use in discerning what people are willing to pay for. It might be possible to use the WTP without QALYs to set limits on health care. WTP per QALY gained, however, should be sidelined. Before updating any cost/QALY threshold, we need to work on what it is that we are asking people they are willing to pay for. Perhaps it will be WTP for a life-year and, separately, WTP for health-related quality-of-life improvements.¹⁶

Regardless of which thresholds are appropriate for limiting health care that’s too costly for what it gains, another consideration is whether we should use different thresholds for different groups or individuals. A paradigmatic example is the end-of-life premium that has gained traction in recent years, particularly in the NHS and its National Institute for Health and Care Excellence.¹⁷ Years of life saved at the end of life are accorded additional value (a premium) beyond their mere number. When the context is a

collective enterprise in which everyone has a stake—an insurance pool, a public provision agency, or standards used widely in the society—even persons who are not elders are likely to become elders and also gain from the premium. An end-of-life premium can thus represent a **wider social judgment** than one favored only by elders. A limited range of such different cost/value thresholds⁹ could make sense if it is simple enough for practical use and the value can be identified with by most members of the pool.

Conclusion

Ethically, WTP per se is not the significantly problematic element in a cost/QALY threshold. The QALY part is. Perhaps, as flawed as it is, a greatly revised version of a cost/QALY threshold would be better.¹⁶ The important focus, however, should be on revising the QALY itself as ethically flawed. The value of a year of life plus the value of quality improvement in a year of life should not be confined to a 1.0 maximum in which the larger the quality adjustment for a condition, the lower the priority for life extension in that condition must be. If the current threshold is simply updated, this more important need will have been ignored.

Dr CXO's answer to a patient or physician should acknowledge that the whole business of discerning a WTP/QALY threshold should be reexamined. The long-term task force should not just establish a new threshold but reexamine the QALY itself.

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CASE AND COMMENTARY: PEER-REVIEWED ARTICLE

How Should Economic Value Be Considered in Treatment Decisions for Individual Patients?

Hadley Stevens Smith, PhD, MPSA

Abstract

Physicians' primary responsibility is to promote patients' well-being, which includes not causing financial harm. Physicians also have duties to prudently steward health care resources. Balancing these responsibilities requires recommending interventions likely to achieve patients' health goals while avoiding unnecessary expenditures. Cost-effectiveness data should be used to inform population-based conceptions of an intervention's value and are not intended to be used by individual clinicians offering recommendations to individual patients. But cost-effectiveness data should be incorporated into patient-clinician conversations about an intervention's affordability and its influence on adherence to a care plan, as these are key promoters of evidence-based practice, value-based care, and optimal outcomes.

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Case

Dr M is giving a lecture on biological therapies for ulcerative colitis (UC), an inflammatory disorder of the colon and rectum known for being expensive to treat and intrusive in the everyday lives of patients. Dr M explains that, for patients with moderate-to-severe disease, UC can be managed medically, since numerous drugs are available. Dr M emphasizes that, although newer biologic drugs seem to be more effective than older drugs, their costs are generally prohibitive. Moreover, newer biologics are not considered cost-effective, although there is wide variation in results of cost-effectiveness analyses for UC drugs, presented in the form of incremental cost-effectiveness ratios (ICERs).

A student in Dr M's lecture asks, "What role should ICERs have in informing decisions about what to prescribe for a patient with UC? I'm trying to reconcile our obligation to recommend evidence-based care while avoiding unnecessary costs to our patients. If the evidence points to a drug that patients can't afford, then how should we think about value-based care for these patients? It seems like most UC patients don't really have

access to value-based care.” Fellow students consider this comment and Dr M prepares to respond.

Commentary

This case considers how medical students should be advised to appropriately factor cost-effectiveness into decisions about individual patient care, specifically in **prescribing decisions** for patients with UC. Discussion of this case considers the following questions: (1) What is the appropriate role of economic value in clinical and ethical assessments of evidence-based care and value-based care? (2) How should cost be used to inform clinicians’ treatment decisions for individual patients? (3) How transparent should modeling details be and to whom should they be made transparent?

Role of Economic Evidence

Physicians’ primary ethical obligation is promoting the well-being of individual patients and, in so doing, they serve as **stewards of health care resources**. Physicians’ patient-level stewardship obligations require that they choose the less costly strategy among alternatives with similar clinical profiles of benefits and harms for individual patients.¹ In aggregate, their treatment choices for individual patients shape overall health care spending and have implications for public health, access to care, health care system quality, and social provision of other essential services. As such, physicians also have a secondary societal-level stewardship obligation to avoid unnecessary or ineffective use of health care resources. Information on the *value* of a particular intervention, such as a pharmaceutical or diagnostic test, defined as “the health outcomes achieved per dollar spent,” is relevant to these stewardship requirements.² Results of economic evaluations, such as societal-level cost-effectiveness analyses (CEAs), together with patient-level evidenced-based medicine (EBM) and value-based care, are 2 approaches to addressing the value of health care.³ Incorporating cost and effectiveness data into physician decision making can serve to advance the related goals of optimal patient care and appropriate stewardship of health care resources at the individual and the societal level.

Cost-effectiveness analysis. CEA involves comparisons of 2 or more interventions in terms of both costs and consequences (effectiveness or benefits),^{4,5} and the results can inform decision making to maximize the efficiency of health care delivery. The result of a CEA is presented as an ICER, which represents the incremental difference in cost compared to the incremental difference in effectiveness of 2 treatment strategies at the population level. Because the analysis reflects outcomes for the average patient, ICERs are not intended to directly guide treatment choices for any individual patient. Instead, ICERs are generally intended to inform decision making for populations, such as payer coverage or reimbursement, and always alongside other relevant ethical, legal, and social considerations.⁴

Evidence-based medicine and value-based analyses. EBM involves the integration of best available evidence, along with clinical expertise and the patient’s preferences, to inform decisions about individual patients’ care.⁶ EBM is primarily concerned with increasing the quality of care for a given patient rather than with cost-effectiveness for health care payers or society.⁷ Value-based analyses can also be applied to individual patient care plan decisions, like EBM, or can be conducted at the level of patient groups, like CEA. Value-based care predominantly emphasizes **quality of patient outcomes**, particularly those that are important to patients. While value-based care shares an outcomes-focused orientation with CEA, the outcomes in value-based care are

summarized not in a single metric, such as a quality-adjusted life year, but rather in a multidimensional set of patient-reported measures.³ Therefore, in the context of the patient-physician encounter, outcomes of interest in value-based care include patient-reported outcome measures that represent various aspects of treatment and well-being, including affordability to the patient.^{2,3}

Role of Cost in Prescribing

The ethically relevant component of EBM and value-based care is the process of shared decision making, which allows patients to express which treatment strategy they perceive as the most valuable.⁸ Physicians should aim for transparency with patients in discussing patients' goals preferences and the potential for cost-related nonadherence. In conversations with patients, physicians' knowledge of the relative economic value of alternative treatment strategies for a given condition can help guide generalized discussions about balancing costs and benefits for various treatment options, with the understanding that the balance might be different for an individual patient than for a population. Promotion of cost conversations between physicians and patients is a key element of initiatives to encourage value-based care, including the [Choosing Wisely campaign](#).⁹

Because patients' perceptions of treatment affordability can figure into their preferences and adherence to a prescribed regimen, conversations about cost are an important part of EBM and value-based care. Cost conversations would ideally integrate data on patients' out-of-pocket costs and the overall economic value of a treatment, for which ICERs can serve as a benchmark, similar to how evidence from clinical research guides discussion of side effects when weighing treatment options.⁸ Awareness of, and engagement in conversations with patients about, trade-offs relevant to treatment choice not only encourages delivery of ethical patient care in [avoiding financial harm](#), but also respects system-level stewardship requirements.

Cost-effectiveness and affordability are not one and the same, however. An intervention that is cost-effective for a population that provides both more health benefits and has a higher cost than an alternative might be considered unaffordable for a given society or health care system if the incremental increase in cost is too high. Moreover, cost-effective interventions that are deemed affordable for populations are not necessarily affordable for individual patients. In the case that a given cost-effective intervention is affordable for both the patient and society, choice of that intervention will be consistent with both individual-level and societal-level stewardship obligations. When these 2 levels of obligations are in conflict, however, physicians' primary responsibility is to the patient in front of them,^{1,10} and prioritization of societal-level spending concerns might be both clinically and ethically inappropriate.^{11,12} EBM's focus on satisfaction of individual patients' preferences, for example, can conflict with a population-based approach subject to social budget constraints.⁷

Although some physicians might perceive cost conversations to be too time consuming and inconsistent with providing optimal care, and physicians and patients alike might be uncomfortable with the topic, evidence suggests that patients want to talk about costs and trade-offs yet rely on their physician to initiate such discussions.¹³ To the extent that the high cost of a medication would inhibit proper adherence, the patient's out-of-pocket cost is relevant information that is not usually otherwise available to physicians, since it is determined in part by the patient's health care payer, prescription drug plan design, and choice of pharmacy. Conversations, then, are crucial to understand the patient's

preferences and concerns related to cost and adherence, particularly for diseases that require high-cost or long-term maintenance medications.

While prioritizing the patient's clinical case and personal values, physicians interpreting ICERs in the context of pharmacotherapy options should be mindful of factors that influence CEA results. As is typical with new interventions, biologics for UC treatment are both more costly than standard care and more effective at increasing quality and quantity of life¹⁴; they are thus considered cost-effective until the ratio of costs to effects is deemed too high (although no firm threshold for this number exists in the United States). Relevant to clinical interpretation of ICERs is the fact that patients' clinical characteristics are a main driver of variation in cost-effectiveness, which can also be affected by other analytic choices.¹⁴

Economic Model Transparency

To allow physicians to properly consider ICERs in the context of providing care for an individual patient, details of model-based analyses should be transparent and accessible to physicians. Data sources for each model parameter value should be clearly presented and justified to enable physicians to assess the characteristics of patient populations included in the data source in light of their own patient's clinical characteristics and goals.^{4,15} Methodologists agree that improvements must be made in communication of CEA results to clinicians to increase efficiency of care delivery,¹⁶ yet there is ongoing debate as to whether making the modeling code publicly available will lead to more meaningful application of results by clinicians, and doing so requires balancing model developers' intellectual property interests.¹⁷

Analytic choices—such as perspective, time horizon, and whether adherence is considered—are relevant to interpretation of CEA results for patient-level decision making. Methodological guidelines recommend reporting results from both a health care sector perspective, which considers medical costs to third-party payers and costs paid out-of-pocket by patients, and a societal perspective, which considers all costs, including patient time cost and lost earnings.¹⁸ For chronic conditions like UC, in which benefits and costs of interventions may differ over a lifetime, the appropriate time horizon of a CEA is the patient's expected lifetime,⁴ and hence a more expensive treatment might also be more cost-effective over the long-term. Differences in patient adherence and thus differences in impacts on health—which might be related to the drug's affordability, the patient's preference for a given route of administration, or the patient's tolerance of side effects—should be reflected in measures of effectiveness and considered over time.

Conclusion

Understanding the appropriate ways in which economic evaluation data can shape physician decision making is crucial to fulfilling individual-level and societal-level stewardship requirements. ICERs provide information on population-level efficiency of treatment strategies, which supports the ethical principle of maximization of health outcomes and can serve to broadly inform clinicians about the value of each therapy as it relates to stewardship requirements. Individual physician decision making based on societal-level spending justifications, however, might be counter to ethical delivery of care for an individual patient. Cost-effectiveness is one form of evidence for consideration in EBM and value-based care, and it should always be used with the ultimate goal of improving an individual patient's outcomes.

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The people and events in this case are fictional. Resemblance to real events or to names of people, living or dead, is entirely coincidental. The viewpoints expressed in this article are those of the author(s) and do not necessarily reflect the views and policies of the AMA.



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MEDICAL EDUCATION: PEER-REVIEWED ARTICLE

How Should Economic Evaluation Be Used to Measure Value and Set Priorities in Health Care?

Sahan Jayawardana, MSc and Elias Mossialos, MD, PhD

Abstract

Novel interventions that are effective and safe but costly suggest the importance of questions about value, accessibility, and affordability. Economic evaluation is one useful tool that health care systems draw upon to help make investment decisions and set priorities. But translating results from economic evaluations into actionable insights about a novel intervention's value remains elusive, given ethical and practical complexities. This article illuminates 5 key factors to consider in interpreting economic evaluations of novel interventions: the health care decision problem, the design and structure of the mathematical model, characterization of uncertainty, awareness of health outcome measure limitations, and the contrast between optimal decision-making conditions and real-world decision-making conditions.

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Economic Evaluation in Health Care

In 2019, the US Food and Drug Administration approved the drug tafamidis as a breakthrough therapy for transthyretin amyloid cardiomyopathy (ATTR-CM) based on a pivotal clinical trial that reported a reduction in all-cause mortality and cardiovascular-associated hospitalizations in patients taking the drug once daily compared with patients who received a placebo over a 30-month follow-up period.¹ However, the manufacturer's wholesale acquisition cost of tafamidis was \$225 000 for 1 year of therapy, making it the most expensive cardiovascular disease drug launched in the United States.¹ Considering the expected substantial clinical benefit of tafamidis, a key question is if it's worth paying this price. Which principles and methods decision makers adhere to in answering this question and in allocating health care resources depends on their preferences about efficiency, equity, universality, and choice.

Economic evaluation is one tool that is widely used to help answer this question.² In essence, economic evaluation aims to characterize the efficiency of health care interventions: How can finite resources be used to maximize the health gains obtained

from them?³ It provides a structured approach to measuring and comparing the health outcomes and costs of competing alternative interventions over time and across populations⁴; to accounting for **individual, payer, or societal preferences**; and to characterizing the inherent uncertainty in model choices and in the precision of model inputs. The results of economic analyses inform decision makers of interventions that could improve the return on resources expended in health care.

An independent economic evaluation estimated that lifetime treatment with tafamidis compared to usual care for ATTR-CM had an incremental cost-effectiveness ratio (ICER) of \$880 000 per quality-adjusted life-year (QALY) gained. The price would have to be reduced by 92% to make it cost-effective at the conventional \$100 000 per QALY threshold.⁵ How does this conclusion translate into actionable insights on value and priority setting in health care? To understand this process, we will illustrate 5 key factors that should be considered when interpreting economic evaluations to support decision making in health care. First, economic evaluations are based on underlying value judgments about the goal of **resource allocation**, which determine how such evaluations can be used to address health care decision-making problems. Second, the design and structure of the economic model used will significantly influence the results. Third, the structure of the model and inputs used to populate it create uncertainty that should be characterized. Fourth, generic measures of health, such as QALYs, have limitations that should be understood when used as an outcome measure. Fifth, there are other factors relevant to health care not accounted for in economic evaluations that should be considered by decision makers.

Understanding the Decision Problem

The theoretical foundation of economic evaluation stems from economic welfare theory, which posits that resource allocation decisions should be assessed based on whether net social welfare is increased.⁶ Applied to health care, this measure of efficiency—known as allocative efficiency—assesses whether the mix of resources being allocated to health care vs the rest of the economy maximizes benefit.⁷ Cost-benefit analysis (CBA) can be used to determine whether an intervention provides an overall net welfare gain and to compare this net gain with that from alternative interventions (see Table), including in other sectors of the economy; implementing the intervention with the greatest net gain will increase efficiency.³ However, CBA is not widely used in health care due to conceptual difficulties in placing monetary value on health or life. The methods widely used in economic evaluation in health care are cost-effectiveness analysis (CEA) and cost-utility analysis (CUA) (see Table), which help assess interventions' productive efficiency in terms of health outcomes gained for a given cost. When interpreting the results from a CEA or CUA, it is important to keep in mind that these analyses cannot indicate whether the amount of health benefits gained from an intervention truly aligns with societal preferences because they cannot compare health care interventions to interventions in other sectors of the economy. In CEA or CUA, a new intervention that is cheaper *and* more effective than alternatives is deemed more efficient. However, like tafamidis, new interventions that are more effective than alternatives generally cost more, in which case, the decision maker must consider the trade-off between health gained and higher cost compared to alternative interventions. The acceptability of this trade-off, given competing priorities, is a core problem faced by decision makers.

Table. Methods of Economic Evaluation

Method	Description
Cost-benefit analysis	Comparison of the costs and outcomes of interventions in monetary terms
Cost-effectiveness analysis	Comparison of the costs of interventions with a chosen primary outcome (eg, cost per case detected)
Cost-utility analysis	Comparison of the costs of interventions with quality-adjusted life-years as the outcome measure

Structuring the Decision

The intuition behind using models to inform economic evaluation is that the consequences of diseases might affect people over several years before manifesting as health outcomes, during which time costs of care accrue. For example, an individual diagnosed with heart failure may experience declining quality of life and hospitalizations and die from cardiovascular or noncardiovascular-related causes. A decision maker would be keen to understand how an intervention, such as tafamidis, would alter these disease events and their cost implications, although some stakeholders, such as insurers, might not be interested in long-term outcomes due to a greater focus on short-term budgetary impact assessment. Modeling enables evidence of the consequences and costs of a disease to be combined and extrapolated over time—often the entire lifetime of a person.⁸

Models, however, are limited by modelers' choices and by data availability. In designing the model, key considerations include the clinical pathways to be included in the model, the availability of data (eg, about the natural history of the disease), and the requisite computational complexity. In addition, the types of health benefits and costs included in the model should be appropriate to the perspective required by the decision maker—eg, the health care system or society. Finally, an important consideration is the representativeness of the clinical data regarding health outcomes. There is evidence to suggest that people of color, women, people with disabilities, and marginalized groups are **underrepresented in clinical trials**, raising concerns about the applicability of trial findings to these populations.⁹ In addition, caution should be exercised when models are used to extrapolate outcomes beyond the period of a clinical trial. The evidence used should be appropriate and systematically chosen.

Characterizing Uncertainty

Uncertainty in economic evaluation stems from 2 sources: uncertainty relating to decisions about, and assumptions inherent in, the structure of the model and uncertainty concerning the precision of the inputs used in the model, such as costs, outcomes, and probabilities of disease events.¹⁰ Structural uncertainty can be explored by examining how alternative scenarios, such as other plausible disease states, events, and pathways affect the results. Parameter uncertainty could be characterized using one-way sensitivity analysis, which involves varying each parameter estimate independently and individually assessing the impact on model results. Additionally, multiway sensitivity analysis could be performed by simultaneously varying more than one parameter (eg, to evaluate model results in a best- or worst-case scenario). More advanced probabilistic approaches such as probabilistic sensitivity analysis (PSA), in

which uncertainty is characterized by randomly sampling model input parameters (eg, costs and treatment effects) using Monte Carlo simulations,¹¹ are now standard practice in health economic evaluation. Characterizing uncertainty in this manner, rather than relying on a single point-estimate of cost-effectiveness, helps make economic evaluation more reliable. However, it is important that the full scope of the PSA be reported, such that all the relevant input parameters are assessed and made accessible to stakeholders.

Basing Decisions on QALYs

CUA, wherein the QALY is used as the health outcome measure, is the most widely used method of health economic evaluation. The QALY combines the quantity of life with health-related quality of life in a single generic composite measure to enable health outcomes to be compared across a range of diseases.¹²

There are ethical problems in using QALYs that require careful consideration. One central criticism in using QALYs to measure health outcomes is that life-years gained by people in full health through a preventive intervention will be considered more valuable than life-years gained by a person who is chronically ill or disabled, with the result that the cost-effectiveness of an intervention may not align with societal preferences.¹³ In addition, common instruments used to measure health-related quality of life, such as the EQ-5D questionnaire, may not adequately characterize the nuanced and complex aspects of disabled health states.¹⁴ Alternative metrics to address some of the limitations of QALYs have been developed, such as health years in total.¹⁵

Perhaps the most controversial aspect of CEA and CUA is the use of the willingness-to-pay (WTP) threshold—a predefined cost per QALY gained above which the intervention would not be considered cost-effective. In practice, it is difficult to accurately identify the outcomes and costs of all interventions in the health care system, so a customary WTP threshold for an additional QALY is assumed as a simplified decision rule. As a result, WTP threshold values have historically lacked an empirical foundation, although implicit thresholds are apparent from the approval decisions of health technology assessment (HTA) bodies.^{16,17} For example, the HTA body in England does not officially identify a WTP threshold above which it would not recommend an intervention, but an intervention with an ICER per QALY gained above £30 000 is generally unlikely to be recommended unless there are other compelling reasons to do so.¹⁸ Similarly, the American College of Cardiology considers an intervention to be of high value if the ICER is less than \$50 000 per QALY gained and of low value if the ICER is greater than \$150 000 per QALY gained.¹⁹ Therefore, thresholds should be considered as indicators of efficiency rather than prescriptive rules. There is likely to be broad consensus that an intervention that costs less than \$50 000 per QALY gained will be reasonably efficient, but one that costs more than \$150 000 will be less so, with exceptions depending on the specific circumstances of the intervention and target population.

Context

Economic evaluation should be viewed as one tool in a decision maker's toolkit to aid pragmatic decision making. Maximizing health gained from available resources should not be the sole objective of a health care system. Other factors that a decision maker must take into account include **equity concerns** in the allocation of health care resources, need, and societal values.²⁰ For example, although the inverse association between socioeconomic status and health outcomes is well-known,²¹ economic evaluations generally do not provide information about trade-offs between cost-

effectiveness and equity. Even within economic evaluation, countries differ in whether they accept QALYs as a measure of health gain.²² Economic evaluation should be used and interpreted with a clear understanding of how it can help decision making. It inherently involves generalizations and simplifications, but these assumptions should be made transparent and explicit to ensure that the model used aligns with the requirements of the health care system, which ultimately should aid decision making that reflects societal values and shared priorities for health care.

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IN THE LITERATURE: PEER-REVIEWED ARTICLE

“Perspectives” in Health Technology Assessment

Anthony J. Culyer, DEcon

Abstract

This article considers 2 types of standard by which health technology assessment (HTA) studies should be judged: methodological and social. Methodological desiderata specify characteristics of a good quality analysis and should be met regardless of context. Transparency about an HTA study’s perspective (eg, specifying whose costs and whose benefits from an intervention should be counted) is one such desideratum. Whether any particular perspective is the right one is, by contrast, contingent upon conditions in which the analysis is to be applied. A perspective ought always to be treated as context sensitive. Recently, it has been advocated that an HTA study’s perspective should always be “societal” (ie, including consequences, good or bad, for anyone affected in any way by a technology’s use). This article argues that this is a mistake, ethically attractive though it might appear.

Health Technology Assessment

Health technology assessment (HTA) is a widely used way of thinking about **setting priorities** in health care investments, selecting treatments for inclusion in or exclusion from insurance benefits packages, or prioritizing the order in which a public health measure is to be rolled out across various population groups.¹ Besides standard textbooks,^{2,3} there are several specific **guides to HTA best practice** in journals.^{4,5,6} It is commonly accepted as good practice for HTAs and related methods like cost-effectiveness analysis and cost-benefit analysis to state the “perspective” from which analyses are to be, or have been, conducted.^{2,3,4,5} *Perspective* defines the kinds of effect, their distributions, and changes in them that are likely to result from health care investment decisions. More specifically, a *societal perspective* was described by Gold et al as one in which “the analyst considers everyone affected by the intervention and counts all significant health outcomes and costs that flow from it, regardless of who experiences the outcomes or costs.”² As examples, among the benefits of a new effective treatment for a chronic disease would be the relief afforded to informal family carers of patients; among the costs of transferring hospital resources from their usual functions to meet urgent needs arising from a pandemic are the health losses associated with the usual treatments no longer available. This article argues that it is a mistake to treat a societal perspective as a general methodological desideratum, ethically appealing though it may appear.

Two Forms of Advocacy

A distinction can be made between *methodological advocacy* and *social advocacy* in the design and conduct of HTA. Methodological advocacy promotes HTA as a tool of analysis with wide applicability and specifies general, context-free standards by which HTA studies may be designed and judged under any decision-making context. Social advocacy in the design and conduct of HTA requires—but does not constitute—the introduction of specific and universal social value judgments, such as the measure of health gain or loss that should be used, the types of consequence that should be considered, the concept of equity that should be used, and the choice of population group that use of the technology impacts. Transparency makes these critically important value judgments clear.^{3,4,5} It is always a desideratum in HTA. It is a universal requirement, regardless of context.

Analysts are also, however, sometimes urged to adopt a particular perspective, usually labelled, as in Gold et al, *societal*. As Drost et al note: “The societal perspective in economic evaluations is important because of its higher decision-supportive power to optimize resource allocation.”⁷ Urging analysts to adopt the societal perspective for the good of society as a matter of procedure is an example of social advocacy. Proponents of the societal perspective include Byford and Raftery,⁸ Johannesson et al,⁹ Jönsson,¹⁰ and Walker et al.¹¹ These authors maintain that taking a societal perspective, as defined by Gold et al, is context-free because it is independent both of the interests of the commissioners of the study, whose interests may well be more limited than a societal perspective, and of the political and social character of the society for which the study is intended.

My suggestion is that all perspectives are context sensitive (ie, appropriate or inappropriate in the context for which a study is intended). The value judgments people hold about health care and its manner of access and delivery vary greatly internationally, and even intranationally, according to circumstance and time and should therefore be treated as context dependent in HTA studies. Specifying a particular perspective, even one as general as societal, ought always to be considered explicitly in each study in the expectation that the context in which the study results might possibly be applied differs from other contexts in ethically important ways.¹¹ There are many cases in which the appropriate perspective may *not* be societal. Examples include studies of workplace health in which decision makers must consider safety interventions conducted from (a) the employer’s perspective, (b) the trade union’s perspective, or (c) a third party payer’s perspective; pharmaceutical interventions assessed from (a) the patients’ perspective, (b) the patients’ caregivers’ perspective, or (c) a health agency perspective; or a family planning intervention assessed from (a) a Roman Catholic perspective, (b) a demographic perspective, or (c) a low-income country’s traditional healers’ perspective. The perspective, in short, ought to match the character of the question any HTA is to address.

As these examples suggest, context includes factors such as disease burden, demography, culture (including religion), traditions, history, wealth, decision-making capacity, data availability, and the degree of risk-aversion in public decision making. It also includes local understandings of health and of **fairness and equity**, the social structures of a society, and the extent to which members of the community in question have shared understandings. In some cases, an HTA might properly adopt the societal perspective, for example, because it is required by a ministry protocol. In practice, however, even health ministries typically adopt a less-than-societal perspective—for

example, by considering only those costs borne by the ministry's budget and those benefits received by insured persons. The choice of perspective is thus rarely context free. Any particular choice is loaded with value judgments¹² and is therefore, as Drummond and Brandt have argued, context sensitive: "the value judgments made in economic evaluations could, quite legitimately, vary from setting to setting."⁴ It is neither necessary nor desirable for all studies to be standardized to take the same perspective, let alone any specific one, but all should state their perspective clearly. Perspective should, in short, always be explicit but also always be context sensitive.

Social Advocacy Assumptions in HTA

Assumption 1: Information costs of HTA can be safely ignored. Conscientiously to search out the most precise estimates of all conceivable costs and consequences of a decision, which is what the societal perspective requires, is to presume that the value of the expected improvement in the quality of the decision in question (somehow measured) is always and everywhere greater than the cost of acquiring the additional information. This presumption is so evidently irrational that it scarcely needs further elaboration. But any compromise on the comprehensiveness of the data set necessarily makes the analysis, to a greater or lesser extent, less than fully societal. A less-than-societal approach is therefore inevitable.

Assumption 2: Analysts make better social value judgments than other people. For analysts to stipulate a specific perspective *for general use* is presumptuous and implies an inherent unearned ethical authority. Stipulating a universal perspective is not a task for which HTA analysts are equipped by technical training, by their ethical rectitude, or by political authority granted through a due process. Analysts are often quite good at eliciting the implicit perspectives and values of decision makers and other stakeholders, which is a useful—indeed, highly desirable—early step in any HTA study, but to *elicit* perspectives and values is not to *stipulate* them. In eliciting them, analysts may also encourage decision makers to reconsider their own presumptions and even to weigh the case for adopting a societal perspective. But the process is not, or ought not to be, one of persuading decision makers to accept the value judgments that happen to be those preferred by the analyst.

Underlying the social advocacy of some analysts is often a specific philosophical view: one that is consequentialist, that is based upon preferences, and that is individualistic. This approach seeks to aggregate the preferences of all individuals in a society over all the possible consequences of the decision in question in order to make a preference ranking. I will not argue against the careful consideration of individuals' preferences, but preferences need to be judged and carefully weighed. (Are ill-informed and well-informed preferences to count equally? Are inconsistent and consistent preferences to count equally?)

Assumption 3: It is ethical to ignore the political, historical, and constitutional contexts of health policy. What is politically acceptable, culturally conditioned, and economically possible varies according to national and regional context. In virtually all jurisdictions, and for reasons well-rehearsed by health economists over many years,¹³ policy and legislative arrangements have been adopted to combat the antisocial consequences of unregulated health care finance and provision: inequity of financial burdens, externalities, imperfect agency, monopoly, transaction costs of insurance, among others. In most jurisdictions, one consequence is the creation of ministries of health with ministers appointed by a due process and accountable—at least in democracies—to a

parliament or generally elected assembly of society's representatives. Governments characteristically set budgets across broad categories of economic activity (eg, health, education, the environment) and also set rules determining how those budgets are to be spent, the consequences to be taken into account in **allocating expenditures**, and the processes of accountability for decisions taken. One conspicuous consequence of these rules is that decision makers in such ministries nearly always adopt a less-than-societal perspective. Two questions therefore demand an answer: is it reasonable when, and by what moral argument do, nonelected, unaccountable, analysts set themselves above elected and accountable public officers? An embarrassingly bold answer to this question was given years ago by a brilliant, but I think misguided, economist of public policy: "the value-judgments made by economists are, by and large, better than those made by non-economists."¹⁴

Analytical Humility

What's wrong with taking one's moral authority from a publicly accountable authority rather than from the preference utilitarianism upon which much of HTA still rests? What's wrong with designing a study according to the objectives set by a client? Why should a study not be designed to tackle only part of a problem? Why should a study not examine consequences for only deprived groups of the population? Why should a study not be designed to identify only the likely losers from a decision (in order perhaps to consider appropriate compensation)? Using HTA to answer these questions requires taking a perspective that can be clearly stated but is not societal.

I recommend analytical humility. As Keynes wrote: "If economists could manage to get themselves thought of as humble, competent people, on a level with dentists, that would be splendid!"¹⁵ That quotation should apply to all analysts—the servants, not the masters—of decision makers.

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STATE OF THE ART AND SCIENCE: PEER-REVIEWED ARTICLE

How Economic Decision Modeling Can Facilitate Health Equity

Jeffrey S. Hoch, PhD, Logan Trenaman, PhD, Shannon M. Hearney, MPH, and Carolyn S. Dewa, PhD, MPH

Abstract

This article offers examples of how modeling can motivate health equity inquiry and research. This article also considers how equity fits into cost-effectiveness frameworks, how economic modeling can broaden the range of options for improving health equity, and how information other than results of cost-effectiveness analyses can inform health technology assessment.

To claim one AMA PRA Category 1 Credit™ for the CME activity associated with this article, you must do the following: (1) read this article in its entirety, (2) answer at least 80 percent of the quiz questions correctly, and (3) complete an evaluation. The quiz, evaluation, and form for claiming AMA PRA Category 1 Credit™ are available through the [AMA Ed Hub™](#).

Cost-Effectiveness Analyses

Cost-effectiveness analysis (CEA) is an important method for determining the relative efficiency of different ways of meeting an objective (eg, maximizing population health). Economic decision modeling creates CEAs for applied health technology assessment processes that make funding recommendations about health care innovations for *populations* (ie, not for a patient or a physician deciding on a treatment). Modeling allows analysts to go beyond the data, extrapolating to different populations using different outcomes over longer time horizons. For example, the Institute for Clinical and Economic Review (ICER) recently evaluated 2 new drugs for the treatment of hereditary transthyretin amyloidosis (hATTR), a rare disease with high unmet need.¹ Using decision modeling, the research team considered the value of the new drugs in terms of both costs and quality-adjusted life-years over the lifetime of patients with hATTR.²

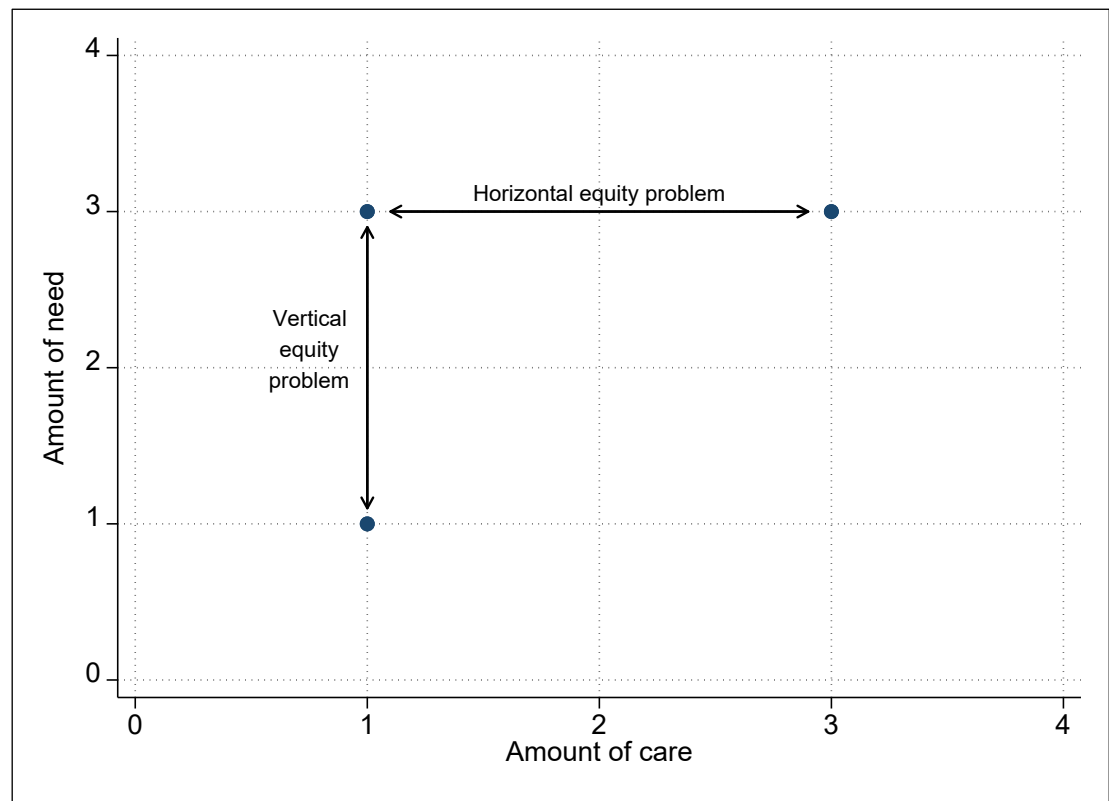
ICER's independent voting panels consider not only cost-effectiveness but also other benefits and contextual considerations, such as social and environmental factors, when assessing the long-term value of new treatments. This process is an inconvenient reality for people hoping for a simple, one-dimensional way to assess value. Recent research by Harvard et al³ reviewed philosophical arguments on the influence of social values throughout scientific inquiry and applied them to better understand where value judgments occur in economic decision modeling. In our paper, we consider practical examples of opportunities for decision modeling to **facilitate health equity** considerations. Our first example considers how equity concerns fit into a standard cost-

effectiveness framework. Next, we consider how economic modeling can broaden the scope of the options for improving health equity by utilizing nonhealth care-related resources. Lastly, we explore the impact on applied health technology assessment of considering other information in addition to CEA results.

Prioritizing Equity in Cost-Effectiveness

In addition to insufficient information to measure the trade-off between efficiency and equity,⁴ another challenge in choosing “reduction of inequity” as the objective in a CEA is that one must define equity (in order to know if it has been reduced).^{5,6} As Gravelle et al note,⁷ the concept of inequity is linked to need and “there is considerable debate about the meaning of ‘need’ and whether equity concerns should relate to health status, the amount of health care received, or access to health care.” After assuming that “need” has been neatly defined, the 2 types of equity challenges in allocating resources are *horizontal inequity* and *vertical inequity*.⁷ Figure 1 illustrates both.

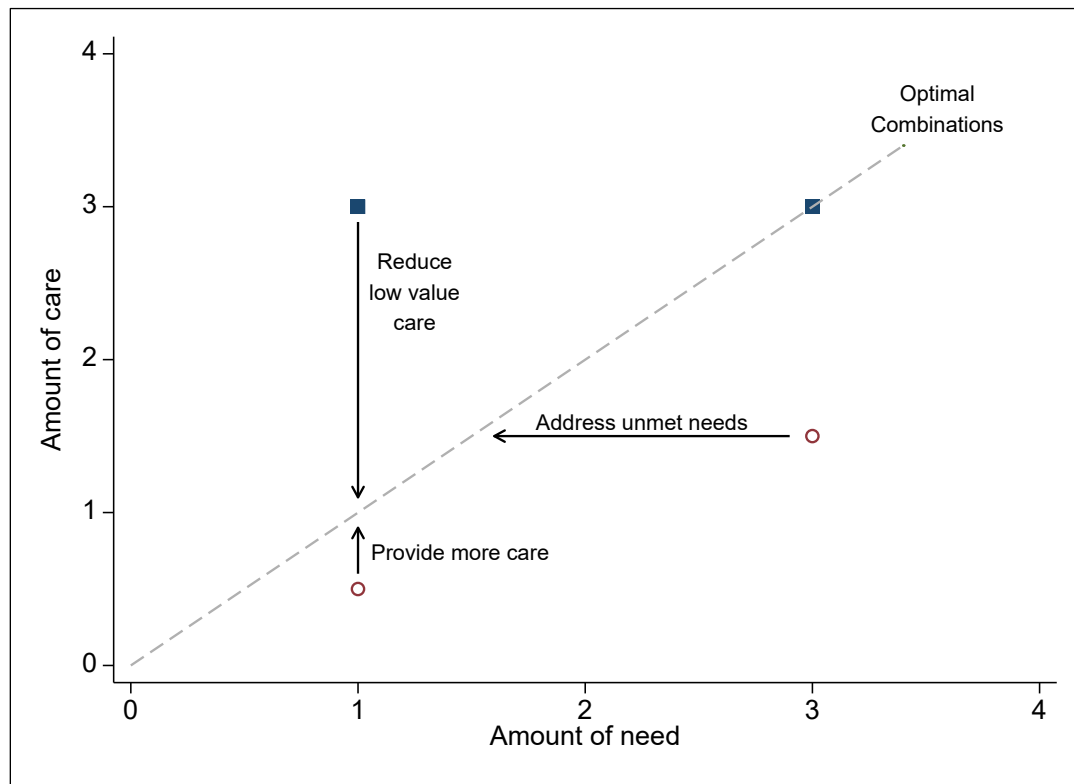
Figure 1. Mismatch Between Care and Need in Vertical and Horizontal Inequity Problems



With horizontal inequity (shown horizontally on a hypothetical scale from 0 to 4, chosen for convenience), people with the same level of need get different amounts of health care (referred to as *care* subsequently). In contrast, with vertical inequity (shown vertically on a hypothetical scale from 0 to 4, chosen for convenience), people with different amounts of need get the same amount of care. It is not clear from Figure 1 in which direction the plotted dots should move to efficiently allocate limited resources. When amounts of care differ (different horizontal position), is inequity more efficiently reduced with more or less care? Likewise, when there are different amounts of need (different vertical position), is inequity more efficiently reduced with more or less care?

Figure 2 presents the optimal combinations of care (illustrated with a dashed line) as a function of need. Figure 2's axes are reversed from Figure 1 so that a higher or lower amount of care is illustrated in an intuitive fashion.

Figure 2. Three Strategies to Address Vertical and Horizontal Inequity Problems



Modeling facilitates health equity considerations by identifying which options should be considered and which should be ruled out. For patients with low need who are receiving too little care (ie, the bottom left \circ in Figure 2), raising the amount of care is optimal. However, the vertical equity problem illustrated by the \blacksquare 's in Figure 2 can be efficiently addressed by actually reducing the amount of low-value care to the level indicated by the dashed line. This observation is a reminder that when optimizing care with respect to need, although it may be commonplace to think first of providing more care to those most in need, addressing the issues of people with *less* need may be both more feasible and more impactful. Interventions **reducing low-value care** for people who have low need might be less expensive (eg, less investment required) and more impactful (eg, more people affected). For low-need patients who are receiving more care than is optimal—such as more tests being ordered than needed—focusing on value instead of volume has the potential to free up scarce resources. Potentially, these resources could then be used to provide additional care for those in greater need. In theory, Figure 2 also illustrates that a more optimal care-need balance for “high-need” patients can be obtained by reducing their needs (instead of or in addition to by providing more care). The dashed optimality line is useful in decision making, as it suggests a way to spend limited resources efficiently to achieve maximum impact. In addition, decision modeling can help determine the dashed optimality line when clinical guidelines include cost-effectiveness information.⁸

Broadening the Option Range

While there are many ways of achieving greater health equity, nonhealth interventions, such as those that address social determinants of health, can be very cost-effective. Indeed, providing housing, food, and safety interventions can be more effective in reducing health inequity than providing more health care. Economic modeling can broaden the scope of the options considered since there is no restriction on which option to study; thus, by examining the value of any option (eg, a social intervention), health equity could be improved. For example, a recent modeling analysis⁹ of options to help homeless individuals considered nonclinical options like housing. When viewed within the framework illustrated in Figure 2, providing housing reduces need (ie, moves people to the left along the horizontal axis). A systematic review¹⁰ of Housing First programs, which provides housing with support services, concluded that while these programs cannot be expected to pay for themselves, “they represent a more efficient allocation of resources than traditional services.” This suggests that the most economically efficient way for a system to cope with homeless people overwhelming emergency rooms may not be to invest in more health care capacity but to reduce need through other social services. Of course, increasing social services will increase costs in a different sector.

CEAs of harm prevention strategies provide another example of how modeling supports the value of nontraditional options for helping vulnerable people. Although harm prevention strategies often involve options that some may find counterintuitive, modeling can show that they are cost-effective in terms of their efficiency in meeting a stated objective. For example, studies of **supervised injection facilities** have found that they are economically attractive,¹¹ especially when the goal is to increase quality-adjusted life-years.¹² However, stigmatization of programs such as these often prevent individuals from considering their cost-effectiveness.¹³

Applied Health Technology

When applied health technology assessment processes incorporate evidence to inform funding recommendations, different values, including health equity concerns, come into play. For example, Trenaman et al¹⁴ reviewed ICER’s health technology assessments and described how cost-effectiveness, other benefits or disadvantages, and contextual considerations affected voting members’ assessments of value. On average, new interventions with more attractive cost-effectiveness results received higher proportions of “high and intermediate” value votes. Among interventions with “other benefits” supporting a product’s value, having a novel mechanism of action (eg, the first generation of a therapy) received the most votes and reducing health disparities received the fewest, possibly because estimates of the health- and nonhealth-related opportunity costs were not available. The results thus highlight that factors beyond cost-effectiveness can lead to lower or higher assessments of value. ICER’s use of economic decision modeling indicates that “reducing health disparities” is an explicit consideration, given its categorization as one of the other benefits to be considered during each value vote. However, the fact that it received a small number of value votes in ICER’s health technology assessments suggests that new health care treatments may not be a common way to remedy health inequity.

Conclusion

Economic decision modeling can facilitate health equity considerations through its design and use.^{15,16} Framing a cost-effectiveness analysis involves focusing on the

structure of the problems and on options, objectives, and costs. Using modeling to study the relative value of different options, including reduction of health inequity, can motivate the “business case” for different types of investments. Modeling allows one to consider different ways of addressing health inequity, which is an important advantage, given that health inequity wounds may be healed by improvements in areas in addition to health care.¹⁷ However, in countries where health care providers seek to maximize profit, one must be able to make the business case that one can do well by doing good. Modeling provides an important input into this process by careful consideration of different options, outcomes, and perspectives. Once the analysis identifies the potential interventions that target social determinants of health, negotiations can begin with a trusted broker to ensure it is worth everyone’s while to aid those who most need help.¹⁸

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POLICY FORUM: PEER-REVIEWED ARTICLE

How Should Economic Analyses Inform Nosocomial Infection Control?

Eleftherios Mylonakis, MD and Panayiotis D. Ziakas, MD, MSc, PhD

Abstract

Nosocomial infections are public health threats with often grave human costs. Because implementing screening and best outbreak response practices is costly for health care organizations, allocating resources for interventions requires consensus among stakeholders with a plurality of perspectives about how to weigh prospective interventions' risks and benefits. Economic analysis can facilitate decision making but is relatively new in nosocomial infection prevention and control. This article describes features of and reasons for economic analysis in this specific area and focuses on emerging challenges in antimicrobial stewardship.

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Nosocomial Infection Costs

Nosocomial infections are a pervasive and costly public health threat. The 5 major health care-associated infections—central line-associated bloodstream infections, ventilator-associated pneumonia, surgical site infections, *Clostridioides* (formerly *Clostridium*) *difficile* infection, and catheter-associated urinary tract infections—add an estimated \$9.8 billion annually in direct US medical costs.¹ If direct, indirect, and societal nonmedical costs are combined, this figure sums up to \$96 to \$147 billion annually.² It has been estimated that, in 2002, approximately 1.7 million patients acquired a health care-associated infection in US hospitals.³ Thereafter, health care-associated infections exhibited a decline,⁴ and later surveys estimated a burden of 721 800 health care-associated infections in US hospitals in 2011.⁵

The notion that **nosocomial infections** are preventable causes of morbidity has led to the development of dynamic programs to control such infections. It has been estimated that up to 70% of catheter-associated bloodstream and urinary tract infections and up to 55% of ventilator-associated pneumonia and surgical site infections could be prevented, thereby saving thousands of lives and dollars spent.⁶ Modern programs incorporate infection prevention policies, surveillance, **outbreak response frameworks**, and antimicrobial stewardship. These practices are endorsed by the Centers for Disease Control and Prevention in its compendium of basic infection prevention and control

guidelines, antibiotic resistance guidelines, and device- and procedure- associated guidelines.⁷ Moreover, these infection control and antimicrobial stewardship programs can be supported by economic analyses and thereby result in cost-effective improvement of care and significant cost savings. In what follows, we will discuss the critical elements of an economic analysis that can shape changes in policies and practices to control nosocomial infections.

Prevention

Economic analysis is crucial to support the development of infection control programs, despite their implementation cost. Nevertheless, economic analysis is a relatively new area in infection prevention programs. Moreover, even when implemented in this context, economic studies are often limited to simple cost analyses and do not adhere to reporting standards.⁸ For example, such analyses frequently report the gross spending per health care-associated infection on the assumption that savings from preventing a high-cost infection will outweigh the extra costs of expanding infection control programs.^{9,10} Additionally, only a minority of cost analyses (weighted average of 6%) are incorporated in medical guidelines,¹¹ reinforcing disregard for cost-saving interventions.

Only a complete cost-effectiveness analysis can safely guide decisions. Such an analysis requires a core of 3 elements: the cost of the new infection prevention policy (in comparison to other interventions), the cost savings from prevented infections, and the clinical benefit.^{12,13} If the measure of clinical benefit includes both duration and quality of life, then a cost-effectiveness analysis is extended to cost-utility analysis, with quality-adjusted life-years (QALYs) being the endpoint. Resulting comparisons of competing strategies are then made based on extra cost per QALY gained instead of extra cost per death averted (or extra cost per infection averted).^{8,14} Thus, relative to alternatives, a new strategy may be less effective and either reduce or increase cost or more effective and either reduce or increase cost. If a new strategy improves outcomes at an increased cost, which is common, a threshold needs to be established to guide decisions by defining an upper limit of spending to gain one QALY (willingness-to-pay threshold).^{15,16}

In the specific setting of nosocomial infections, the extraction of cost savings resulting from prevented infections is particularly challenging, especially for seriously ill patients who are already receiving high-cost care, regardless of their infection status. Moreover, apart from direct program implementation costs, there are collateral costs related to suboptimal treatment for nosocomial infections, drug adverse reactions, and the emergence of **antibiotic resistance**, all of which must be included in cost-savings calculations.¹⁷ Additionally, infection prevention policies are unique in the sense that reducing prescribing of one class of antibiotic may be counteracted by prescribing of and emerging resistance to another class of antibiotic (known as “squeezing the balloon effect”),^{18,19} which might compromise infection control plans and increase associated costs when antimicrobial restrictions are implemented.

Given these considerations, it is often impossible to define the most accurate values for the input variables. While the baseline assumptions can be considered “a best guess,” a sensitivity analysis over a range of input variables is necessary to determine the robustness of conclusions, as exemplified by methicillin-resistant *Staphylococcus aureus* infection prevention strategies in intensive-care units.²⁰ Unlike clinical studies, cost-effectiveness analyses until recently lacked a standardized guideline for how they should be conducted and reported. The publication of the Consolidated Health

Economic Evaluation Reporting Standards guidelines,²¹ along with earlier influential recommendations,^{22,23} has provided the framework for analysts to report the key elements of cost-effectiveness analysis, and these guidelines have been adopted in recent studies on health care-associated infections.^{24,25}

Facilitating Decision Making

One challenge in economic analysis and in decision making is the differing perspectives of stakeholders. Clinicians focus mostly on the effectiveness of an intervention and less on the monetary cost. Health care administrators aim to **optimize the allocation of resources** and, in collaboration with clinicians, to improve health outcomes. For example, although it might be easy for administrators to adopt a universal influenza vaccination program for health care workers,²⁶ in an era of increasing health care complexity (and cost) and constrained budgets, more complex decision making can be a challenging task (see Table).

Table. Decision-Making Goals and Challenges for Health Care Organizations

Goal	Challenge
Fiscal resource stewardship	Optimize anticipated returns from limited resource investments. Manage risks of implementing cost-effective interventions that might not be affordable.
Balance different perspectives	Stakeholders have different, sometimes conflicting priorities (ie, organizations focus on cost-benefit analysis, clinicians and patients focus on effectiveness).
Manage lag between implementation and benefit	New interventions have immediate implementation cost but delayed and ambiguous benefits.
Adopt guidelines	Few guidelines adopt economic analyses to support intervention policies.
Manage emerging threats	Outbreaks and epidemics magnify risk aversion tendencies and preclude conducting valid economic analyses early.
Manage fiscal uncertainty	Account for uncertainty about whether pay-for-performance goals will be achieved by interventions implemented.
Forge consensus	Although necessary to optimize decisions, collaboration and forging consensus among policymakers, clinicians, epidemiologists, and other experts might be difficult.

Economic analyses need to focus on costs important to administrators and third-party payers, a task made more challenging by the fragmentation of care. Hospital administrators are interested primarily in costs paid by the hospital, not by the patient. Thus, they are unlikely to support costly interventions that may reduce nosocomial infections but have a worse cost-benefit profile than alternatives, as other hospital functions would be deprived of critical budgetary resources. They opt for a new strategy when it has a large incremental effect and a smaller incremental cost. Even when a cost-effectiveness analysis favors a new intervention, it may be rejected as unaffordable if it pertains to a large inpatient population and another intervention would have minimal impact on the nosocomial budget. From a regional or national perspective, costs have a wider definition, as they include hospital costs, out-of-pocket patient costs, and societal costs. Societal costs include health care utilization, time to seek care, outpatient caregiving, and loss of economic activity.

There needs to be a balance between proven and new strategies in using limited resources to maximize health outcomes. For example, the clinical best care practices to prevent infections, such as hand hygiene, sanitation, and screening, are not questioned and are considered cost-effective. The pertinent savings from such measures can be used to fund human resources, medical equipment and materials, information technology,²⁷ or antimicrobial stewardship programs.²⁸ However, administrators can be reluctant to introduce new interventions, particularly if there is an additional cost without a clear short-term benefit. A new intervention is associated with immediate additional spending on workforce or equipment, while the perceived benefits for an administrator and hospital may arise several years later owing to reductions in morbidity and mortality.^{17,29}

Decision making becomes even more challenging in the setting of outbreaks, either at the local level or during a pandemic, such as the one brought about by the novel coronavirus (SARS-CoV-2).³⁰ High variability and uncertainty of input values arises when defining the economic analysis model. Many unknown components are involved in a new setting—namely, transmission rate, mortality rate and outbreak expected duration, novel surveillance detection tests, and new or repurposed drugs. An economic analysis may not be feasible to guide early decisions, and administrators and policymakers might be more disinclined to take risks.^{31,32,33} To make things more complex, during an outbreak, if multiple interventions are introduced at once, conclusions about each intervention's effectiveness are confounded. As more data become available, revision of implemented strategies can enable selection of more cost-effective strategies.³⁰

Nevertheless, there are hospital infection prevention policies that are nonnegotiable, regardless of any economic analyses. For example, nuclear acid testing of blood products to cover the window period for HIV infection is far from cost-effective,³⁴ but it is universally required for the zero-risk transfusion practice demanded by society. Moreover, the Centers for Medicare and Medicaid Services ties reimbursement to quality benchmarks using financial incentives (and disincentives), including the Hospital-Acquired Condition Program that comprises 5 major nosocomial infections: central line-associated bloodstream infections, surgical site infections, *Clostridioides* (formerly *Clostridium*) *difficile* infections, catheter-associated urinary tract infections, and methicillin-resistant *Staphylococcus aureus* infections. These infections weigh significantly in hospital reimbursement, and failure to achieve the established goals results in harm to the patient and additional financial cost to the hospital. In other

words, hospitals face both the burden of nosocomial infections and the added financial risks through **pay-for-performance systems**.³⁵

Conclusion

In conclusion, quantitative and qualitative improvement of economic analysis in the field of nosocomial infection control would facilitate administrators' and policymakers' timely adoption of effective solutions and improve resource allocation for the benefit of health care organizations, physicians, and patients. Close collaboration among administrators, infection control experts, epidemiologists, and those with economic evaluation expertise is necessary to merge the evolving evidence-based guidelines with cost-effective platforms of interventions in a highly competitive health care environment. Such integration across the continuum of health care might optimize the quality of patient care, improve health outcomes, and contribute to patient satisfaction.

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POLICY FORUM: PEER-REVIEWED ARTICLE

How Does Cost-Effectiveness Analysis Inform Health Care Decisions?

David D. Kim, PhD and Anirban Basu, PhD

Abstract

Cost-effectiveness analysis (CEA) provides a formal assessment of trade-offs involving benefits, harms, and costs inherent in alternative options. CEA has been increasingly used to inform public and private organizations' reimbursement decisions, benefit designs, and price negotiations worldwide. Despite the lack of centralized efforts to promote CEA in the United States, the demand for CEA is growing. This article briefly reviews the history of CEA in the United States, highlights advances in practice guidelines, and discusses CEA's ethical challenges. It also offers a way forward to inform health care decisions.

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Background

Decision makers in health care often face challenging questions. Should clinicians check every adult's blood pressure? Should a health plan's drug formularies cover a new and expensive drug? How should vaccines or treatments be priced in a pandemic? Answers to these questions require careful examination of potential trade-offs involving benefits, harms, and costs associated with policies or health interventions to determine the optimal choice.¹

One approach to aid such decisions is to conduct a cost-effectiveness analysis (CEA) that explicitly quantifies the relative costs and benefits of alternative interventions.^{2,3} It aims to illuminate the potential trade-offs and inform discussions of whether the additional resources demanded by an intervention (over an alternative) are worth the additional gain in health produced by it.⁴ A CEA expresses this trade-off using a metric called the incremental cost-effectiveness ratio (ICER).⁵ The ICER can be regarded as a "price" for an additional unit of health gained through an intervention. Like lower prices, a

smaller ratio is more favorable because it implies that an intervention can produce an incremental health gain at a lower cost. ICERs are often compared to a range of predetermined threshold values that reflect the willingness to pay for an additional unit of health gain from the perspective taken. For example, the willingness-to-pay threshold usually ranges from \$100 000 to \$150 000 per additional unit of health gain measured by quality-adjusted life-years (QALYs) in the United States.^{6,7,8} It implies that if the ICER for the intervention lies below the chosen threshold, it is deemed cost-effective.

Since the 1990s, the number of CEAs has grown substantially, covering a wide range of diseases and interventions.^{9,10,11} In the United States and abroad, many public and private organizations have formally adopted a health technology assessment (HTA) process that uses ICERs to inform reimbursement decisions, benefit designs, and price negotiations.^{12,13} The rest of this article offers a brief on the use of CEA in US health care, highlights advances in CEA practice guidelines, discusses ethical challenges of using CEA for health care decisions, and suggests a way forward.

Cost-Effectiveness Analysis in the United States

Unlike many other developed countries where CEA has been incorporated into the formal HTA process,^{11,13} the United States has resisted following suit. One exception is the use of cost-effectiveness evidence by the Centers for Disease Control and Prevention's Advisory Committee on Immunization Practices to inform national recommendations on immunization policy.¹⁴ However, a growing concern about inefficient health care spending has led to the incorporation of value (typically measured by ICERs using QALY as the measure of health gain) into organizations' health care decisions and practice guidelines.^{15,16} For example, the Institute for Clinical and Economic Review (ironically, with the moniker ICER), a US-based nonprofit organization, applies systematic and evidence-based approaches—including CEAs—to assess the value of various health technologies.⁷ Medical professional societies and other organizations have also developed practice guidelines incorporating value measured by ICERs, for example.¹⁷

Nevertheless, the use of cost-effectiveness evidence to inform health care decisions faces challenges and opposition from policymakers, the drug industry, and patient advocates. Our fragmented health care system with its various key players diminishes the incentive to consider the broader implication of **resource allocation decisions**.¹⁸ Some resistance to CEA pertains to Americans' aversion to rationing and unwillingness to accept limits in the delivery of health care.^{19,20} Methodological challenges—often based on incomplete evidence (eg, the need to extrapolate clinical trial data beyond follow-up periods), CEA's limited applicability in assessing effectiveness in the real-world setting, and judgment calls made by the analyst—have contributed to the mistrust of results.^{20,21,22} Although how well and how widely CEA will be accepted and implemented in the United States remains to be seen, CEA methods have been substantially improved to meet some of these challenges.

Advances in Cost-Effectiveness Analysis

In 1996, the US Public Health Service’s Panel on Cost-Effectiveness in Health and Medicine established a reference case analysis, a set of standard methodologies to improve the quality and comparability of CEAs that emphasized using QALY as a health outcome measure and applying a **societal perspective**.² A QALY measures the value of health gains as a function of both being alive and the quality of health captured by health-related quality-of-life weight.²³ QALYs enable comparison of well-being related to health across patients, diseases, and treatments—a necessary step to inform resource allocation decisions. The panel also endorsed a broader societal perspective because considering everyone affected and counting all benefits and costs, regardless of who gains or loses, can provide the basis for fair decisions in the public interest.^{2,24} The societal perspective accounts for disease and intervention-related nonhealth impact, including patient time, patient transportation, unpaid caregiver time, productivity loss, and spillover impact on other sectors, such as education.¹⁰

Twenty years later, the Second Panel on Cost-Effectiveness in Health and Medicine was convened to provide an updated guideline reflecting methodological advances in evidence synthesis, modeling, uncertainty analysis, and consideration of ethical and distributional issues.³ The table summarizes reference case analyses of the field’s major guidelines. One of the second panel’s significant updates was the recommendation of 2 reference case analyses: one from a health care sector perspective and another from a societal perspective.²⁵ The second panel recognized that the use of a societal perspective had declined since 2000 while a narrower health care sector or payer perspective persists in most published CEAs.¹⁰ The failure to apply a societal perspective might reflect (1) difficulties in defining nonhealth impact (eg, lost productivity or reductions in resource consumption) associated with a particular disease or an intervention, (2) absence of available data to quantify nonhealth impact, and (3) international guidelines that endorse a narrower health care sector or payer perspective, which may better represent the interests of particular budget holders who are not generally concerned about nonhealth impact that falls outside of their budget.

Table. Comparisons of Reference Case Analyses in Cost-Effectiveness Analysis Guidelines

Elements of analyses	Reference case analyses				
	1st US Panel (1996) ²	UK NICE reference case (2013) ²⁶	Gates/iDSI reference case (2014) ²⁷	2nd US Panel (2016) ³	ICER value assessment framework (2020) ⁷
Perspective	Societal	National Health Service	Health care sector	Societal & health care sector	Societal ^a & health care sector ^b
Comparators	Available & feasible options (including existing practice and a do-nothing option, as appropriate)				
Measurement of health effects	QALYs gained	QALYs gained	DALYs averted	QALYs gained	QALYs gained & equal value life-years gained

Time horizon	Long enough to capture all relevant future consequences (eg, lifetime)				
Discounting ^c	3.0%	3.5%	3.0%	3.0%	3.0%
Evidence	Use of all relevant evidence (eg, systematic and transparent approach)				
Nonhealth impact ^d	Should be included	If relevant, should be identified and reported separately	If relevant, should be identified & reported separately	Should be included	Should be included in scenario analysis for its modified societal perspective

Abbreviations: DALYs, disability-adjusted life years; ICER, Institute for Cost-Effectiveness Research; iDSI, International Decision Support Initiative; NICE, National Institute for Health and Care Excellence; QALYs, quality-adjusted life years.

^aThe Institute for Comparative Economic Research recommends a modified societal perspective analysis as a scenario analysis. However, when the societal costs of care for any disease are large relative to the direct health care costs and the impact of treatment on these costs is substantial (ie, there are substantial differences in the cost-effectiveness findings between the 2 perspectives), the societal perspective is included as a co-base case, presented directly alongside the health care sector perspective analysis.

^bPrimary base case.

^cCosts and benefits.

^dNonhealth impact refers to nonhealth effects of disease and its treatment, including patient time, transportation costs, caregiver time, productivity, and other nonhealth care sector impacts on education, criminal justice, housing, and environment.

Nevertheless, the second panel recognized the importance of capturing nonhealth impact that matters to the broader society, recommending that analysts attempt to quantify nonhealth impact and include it in estimates of value (eg, ICER) unless the effect is likely negligible.²⁵ The second panel advised the use of an “impact inventory,” a structured table listing an intervention’s health and nonhealth impact, to improve analyses conducted from a societal perspective.²⁵ The structured table can signal to audiences which health and nonhealth effects have been included in or excluded from analyses and whether analyses are likely to under- or overestimate the value of an intervention. For example, when analyzing policy and health interventions related to the COVID-19 pandemic, accounting for nonhealth impact is essential for generating reliable and comprehensive estimates of an intervention’s full value.²⁸

Ethical Considerations

There have been a few criticisms on ethical grounds of CEA’s use for decision making. These include (1) controversies associated with the use of QALYs, (2) distributive justice, and (3) incomplete valuation. We discuss each of them in detail here. However, it is worth pointing out that cost-effectiveness evidence is only one of many factors considered in resource allocation decisions. We have found that none of the international HTA bodies bases its decisions solely on cost-effectiveness evidence. Therefore, much of CEA’s criticisms, fair or not, can be addressed through deliberative processes.²⁹

QALYs. The lower health utility, or health-related quality of life, assigned to patients with worse health (because of more severe disease, disability, age, and so on) raises distributive issues in using QALYs for resource allocation decisions. For example, because patients with disabilities have a lower overall health utility weight, any extension of their lives by reducing the health burden from one disease “would not generate as many QALYs as a similar extension of life for otherwise healthy people.”³⁰ This distributive limitation arises because of the multiplicative nature of QALYs, which are a product of life-years and health

utility weight. Consequently, the National Council on Disability has strongly denounced the use of QALYs.³¹

Alternatives to QALYs have been proposed. The Institute for Clinical and Economic Review has started using the equal value of life-years gained metric,³² a modified version of the equal value of life (EVL) metric,³³ to supplement QALYs. In EVL calculations, any life-year gained is valued at a weight of 1 QALY, irrespective of individuals' health status during the extra year.³⁴ EVL, however, "has had limited traction among academics and decision-making bodies" because it undervalues interventions that extend life-years by the same amount as other interventions but that substantially improve quality of life.³⁰ More recently, a health-years-in-total metric was proposed to overcome the limitations of both QALYs and EVL, but more work is needed to fully understand its theoretical foundations.³⁰

Distributive justice. The second criticism pertains to the fundamental notion that "a QALY is a QALY is a QALY no matter who gets it."³⁵ Because of this egalitarian notion, the question of whose values shall count for how much raises some ethical issues. For example, should large benefits to a small number of people receive priority over smaller but greater aggregate benefits to a large number of people? Or when should society give priority to treating the sickest or worst off?^{36,37,38,39,40} However, CEA was not meant to address such **distributional considerations** directly. The Second Panel on Cost-Effectiveness in Health and Medicine emphasized that such distributive considerations also matter to decision makers and are often part of deliberative processes.^{3,4} Areas of ongoing research include the development of equity weights, which assign numerical values based on considerations other than QALYs (eg, the severity or rarity of the disease), and incorporating social distributions of health (eg, by income or ethnicity) into CEA.^{41,42,43}

Incomplete valuation. The third criticism relates to CEA's consideration (or lack thereof) of certain value elements. Many HTA bodies around the world use CEA from a health care sector perspective and do not incorporate value elements such as productivity, time costs, caregivers' costs, and spillover to other sectors of the society.¹⁰ Even in the United States, ICER has not considered these elements formally, although more recently it has allowed for a modified societal perspective as a secondary analysis.⁷ The Second Panel on Cost-Effectiveness in Health and Medicine, which recommends analyses using both the health care sector perspective and the societal perspective, has laid out the methods for incorporating such value elements.³ Often, lack of data (eg, the effect of a treatment on productive time) precludes analysts from including some of these value elements in the analysis, even though they are generally believed to be important to patients and their caregivers. Although recent advances in measuring these value elements have provided a set of useful resources,^{44,45} more work is needed to readily incorporate these elements into standard CEA.

Informing Decision Making

Resources to improve health are always limited. It is impossible to provide all the interventions that offer health benefits without sacrificing resources that could be used for other desirable and important goals, such as education.

Consequently, whether explicitly or implicitly, some form of prioritization or **rationing is unavoidable**. Without considering opportunity cost, we would not know whether better use of those resources was possible; choosing an intervention in ignorance of opportunity costs cannot be deemed ethical, either. Although it does not capture all relevant concerns, CEA is a systematic and explicit way of assessing a given decision's opportunity cost.

In the United States, a step forward would be the establishment of a national HTA agency that formally incorporates cost-effectiveness evidence along with other contextual elements, such as distributional concerns and budget impact. With a transparent, scientifically rigorous, and deliberative process of assessing trade-offs among alternative health policies or interventions, such an agency could provide valuable information to better inform resource allocation decisions in health care, including value-based prices for price negotiations.

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POLICY FORUM: PEER-REVIEWED ARTICLE

How to Motivate Equity in Health Decision Modeling

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Abstract

American health care is rife with inequity in access to services. Even among people with insurance, inequity can result from insurers' decisions about which services to cover. These decisions are often based on economic models that are seemingly objective but neglect factors affecting people who are economically disadvantaged. Laws and government programs designed to mitigate inequities in access have limited value in addressing bias in models that inform coverage decisions. As a reform, government agencies that fund research could require that studies on which decision models are based better account for factors affecting people who are economically disadvantaged, an approach this article explores.

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Introduction

Health care demand may be unlimited, but health care resources are not. Even in a system that costs more than \$3.8 trillion a year in the United States,¹ it is impossible for everyone to get everything they need. For consumer goods, the market determines how much an individual can obtain by setting a price. However, health care is different. It is essential to life and well-being, so denying it to those with fewer resources raises ethical concerns about inequity in the form of unfairness generated by unequal treatment.²

This article describes health care inequity that can result from biases that seep into economic models on which coverage determinations are based. It also considers limitations of current laws and government programs that seek to **mitigate inequity in health care** access and suggests a reform for creating economic models that better promote health equity. Mitigating bias in economic modeling would not eliminate all US health care inequity, but it would help to illuminate actual needs, improve resource allocation decisions, and promote greater justice and beneficence in the system.

Access Inequity

American health care relies heavily on market mechanisms, leading to inequitable access to many services based on ability to pay.³ Despite the coverage expansion under the Affordable Care Act (ACA) of 2010,⁴ almost 10% of the US population remains uninsured.⁵ Even patients with insurance may face unaffordable deductibles and copayments for services.⁶ A health system with wealth-based impediments to access violates the ethical principles of justice, which calls for fair allocation of resources, and beneficence, which calls for helping those in need.

Insurers' decisions about which services to cover can introduce another source of access inequity. Such decisions are often based on economic modeling, which quantitatively assesses costs and expected benefits of new interventions.⁷ Decision models may appear objective, but when they fail to account for factors affecting economically and socially disadvantaged populations, they can promote bias in coverage.

Can law help to mitigate this latter source of inequity? There are laws that prohibit some forms of discrimination in the provision of health care services, and there are government programs, such as Medicaid, that extend coverage to resource-poor members of the population. However, these laws and programs have limited usefulness in combatting bias built into coverage determinations. More effective means are needed to make economic modeling more equitable.

Better Modeling

Assigning dollar amounts to costs in an economic model is generally straightforward, but benefits are more difficult to measure. Calculation methods can introduce biases in coverage determinations in several ways, two of which are considered here.

Subjectivity in benefit measurement. Measurement of benefits gained from an intervention usually requires making subjective judgments⁸ concerning, for example, the extent to which pain and suffering caused by a condition will likely be reduced, the number of quality-adjusted life-years (QALYs) expected to be gained from an intervention, and the level of functional capacity expected to be gained or regained. QALYs reflect the strength of individual preferences for health states (eg, perfect health)⁹ that can vary with patients' economic and social circumstances.¹⁰ Failure to account for such variation can create or exacerbate inequity.¹¹ For example, a model comparing the cost-effectiveness of a new intervention to an older one that is more invasive and costly might assume that an underlying condition treated by the new intervention causes only minimal discomfort when administered at home. But for **patients lacking access** to nutritious food or safe housing, home care might be difficult or impossible, so achieving "minimal discomfort" in a home environment might not be equitably accessible to all. In this case, the intervention would be more valuable for people with compromised access to food and housing if it were administered in a clinical setting.

Subject selection. Clinical trials that evaluate an intervention's safety and efficacy might not be equitably designed, especially in terms of **subject recruitment**. Demographically homogeneous subject samples, with inadequate representation of members of racial or ethnic minorities, women, or the elderly, for example, can introduce a source of bias.¹² This bias can influence conclusions drawn about an intervention's safety and efficacy in these groups. If an intervention is approved, its value for the kinds of people who were

not adequately represented in its clinical trials might be underestimated when insurers use models informed by such research to make coverage determinations.

Reform

Several laws help to mitigate inequity in access by prohibiting discrimination in health care service provision. Title VI of the Civil Rights Act of 1964 prohibits discrimination based on race, color, or national origin by recipients of federal funding (including health care organizations),¹³ and the Americans with Disabilities Act of 1990 prohibits discrimination based on disability in most public services, including health care.¹⁴ However, because these laws have limitations when it comes to indirect discrimination, such as bias resulting from economic models that guide insurers' coverage determinations, such violations would be difficult for a plaintiff to prove.

In addition to Medicare and Medicaid, the ACA facilitates Americans' access to private health insurance policies if they are unable to obtain coverage elsewhere, and it subsidizes premiums for people with low incomes.⁴ However, decisions about which interventions insurers cover under the ACA are still subject to the potential bias discussed above. Legal or regulatory approaches to eliminating this bias by addressing decision modeling methods would be difficult to implement, as they would require oversight of proprietary analyses. They would also have to navigate constraints imposed by other federal laws that govern insurance, most notably the Employee Retirement Income Security Act.¹⁵

Leveraging the federal government's roles in funding research that decision models draw upon might be an easier approach. Agencies that fund research, such as the Agency for Healthcare Research and Quality,¹⁶ the Patient-Centered Outcomes Research Institute,¹⁷ and the National Institute on Minority Health and Health Disparities within the National Institutes of Health,¹⁸ could require that studies forming the basis of decision models better account for factors that affect people who are economically disadvantaged.¹⁹ In addition, the Centers for Medicare and Medicaid Services, the agency that administers Medicare and Medicaid, could require that coverage decisions under these programs be informed by decision models that appropriately consider their interests.

Among the most important factors to include in the development of decision models to reduce inequity are those affecting social determinants of health and public health.²⁰ Social determinants include economic, environmental, and social factors that influence health risk susceptibility and the likely benefits of treatment.²¹ For example, residents of neighborhoods with polluted air might be more susceptible to asthma and more vulnerable to its recurrence, and residents of neighborhoods with more crime might be more susceptible to conditions caused by stress, such as heart disease. Public health, which views health through a population rather than individual lens, is especially important for members of economically disadvantaged groups who have limited access to health care services.²⁰ Thus, it is key to promoting equity.²² Economic models that consider social determinants and public health would make inequitable coverage decisions less likely.

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ART OF MEDICINE

American Value

Kelsey Coolahan

Abstract

A canvas of hand-carved woodblock prints in red ink and gold acrylic lettering offers a visual representation of physicians' impulses to practice healing in the American health care sector.

Figure 1. *Review of System*



Figure 2. Detail from *Review of System*



Media

Hand-carved woodblock prints in red ink on 5 x 4 ft cloth canvas with gold acrylic lettering.

Caption

The approximate, annual per capita cost of health care in the United States is represented by a hundred blood-red \$100 bills. The bills also represent the multibillion dollar business of health care—the corporate mergers, private equity firms, and Silicon Valley entrepreneurs—that is **shaping the health care landscape** for better or for worse. Gold lettering in the background reads: “All other systems were reviewed and are negative.” This phrase, often auto-populated in clinician notes for billing and legal purposes, brings to mind the bureaucratic duties and constraints placed on American physicians. The gold font nods to the abandoned monetary system of the gold standard. This piece serves as a call to action for physicians and policymakers to review and address the driving forces behind today’s American health care system.

Kelsey Coolahan is an MD/MBA candidate at Cooper Medical School of Rowan University in Glassboro, New Jersey. Her interests include health equity, medical education reform, and physician activism.

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ART OF MEDICINE

How Valuable?

Dahlia Fischer

Abstract

A wire, plaster, and wood sculpture of a DNA double helix with one mismatched base pair considers how we might justly weigh overall health equity against extremely costly interventions for uncommon genetic illnesses.

Figure. *Is the Price Right?*



Media

Wire, plaster, and wood.

Caption

One mismatched base pair in a DNA sequence can cause fatal illness in its carrier. Interventions continue to evolve, offering promise for healing, perhaps, for some patients with some genetic illnesses. Prospective somatic or germline approaches are ethically fraught when they can help few, when their **high costs** make them inaccessible to most, and when safety and efficacy for individuals and their descendants is unknown or, perhaps, unknowable. This work asks a viewer, “How should value be assessed, and according to whom?”

Dahlia Fischer graduated from the New York University College of Arts and Sciences with a major in psychology and a minor in studio art. In her artwork, she explores various themes and their interconnection and representation in the world.

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ART OF MEDICINE

An Animated Portrait of Inaccessibly High-Cost Care

Taylor Colette Moon, MFA

Abstract

This digital still and the poetically narrated animation portrait from which it is captured characterizes a woman overwhelmed by her body's failure and by a health care system's failure to care well. Her story offers insight into a patient's experience of inaccessibly high-cost services.

Figure. *Walk With Me*



Media

Digital still of an animated portrait.

Caption

The **animated portrait** begins with a woman in a hospital cafeteria (see Figure) looking for someone to walk with her to her chemotherapy appointment. Overwhelmed by her **terminal diagnosis**, sadness in her countenance swells as she tells her story, “And I told the doctor, ‘I think there is something wrong.’” This remark is the point when her body informs her of her condition. She resists taking pain medication to be present with family members and spends money on better food instead of medicine.

Taylor Colette Moon, MFA is an assistant professor of graphic design at Missouri Southern State University in Joplin, Missouri, and a contemporary artist whose primary medium is poetically narrated animations. She received a bachelor of arts degree from the University of California, Santa Barbara, with a double major in art and art history, and thereafter received a master of fine art degree from the University of Oxford’s Ruskin School of Art.

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ART OF MEDICINE

Ironic Currency

Kashvi Gupta, MBBS, MPH, Kehaan J. Saraiya, and Saurabh Jha, MD, MS

Abstract

A 3-panel comic illustrates an offer of data to pay for health care services.

Figure. *Is Big Data the Currency of the 21st Century?*



Media

Digital illustration.

Caption

Availability of large amounts of many patients' personal and health data has made some **artificial intelligence applications in health care** possible and has enabled growth in personalized medicine. Big data's capacity to divert attention from a patient's care might be ironic, then, when considered in light of its overwhelming value to organizations competing in a health care marketplace with few cost controls.

Kashvi Gupta, MBBS, MPH is an incoming first-year resident at the University of Missouri-Kansas City. She earned her MBBS degree from Kasturba Medical College in India and her MPH degree in global health epidemiology from the University of Michigan. Her research interests include biomedical ethics, cardiovascular medicine, and global health.

Kehaan J. Saraiya is an artist and the founder of the Blue Matchbox Studio in Bangalore, India. His creative pursuits and curiosity have led him to work in various fields of art and design. The Blue Matchbox Studio hosts exhibitions, workshops, talks,

musical events, and much more to build a local community of creative individuals in Bangalore.

Saurabh Jha, MD, MS is an associate professor at the University of Pennsylvania in Philadelphia. He's a prolific writer and a budding artist.

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AMA Journal of Ethics®

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PERSONAL NARRATIVE

Set Down the Stethoscope

Michael Westerhaus, MD, MA

Abstract

In this moment of pandemic and social unrest, health workers feel overwhelming exhaustion, uncertainty, and powerlessness. At the same time, a deep spirit of resilience and a desire for innovation, discovery, and justice compel health workers to retain their commitment to serving patients and communities. This essay takes this context into consideration and proposes pragmatic steps toward a reimagined and reinvigorated future for our work as health workers.

Learning the Art of Patient Distance

Long before a barbed virus would so thoroughly upend our lives, I bought my first stethoscope in 2001. With this symbol of tremendous power around my neck, I sought a career of proximity to patients' lives. Proximity, however, wasn't something I would learn. My first lessons in medical school were with cadavers and pathology slides. When I started conversing with patients, my teachers handed me a formulaic script that largely avoided their personal lives and social context. In residency, I learned to provide more patient care in less time and that sharing who I was with patients was risky and unprofessional. I absorbed that medical care happens on our terms in our spaces; outside of 3 home visits, all my patient encounters were inside hospitals and clinics.

Learning the Art of Personal Distance

I learned to auscultate by listening to the hearts of classmates, patient-actors, and, eventually, patients. Curiously, never did I learn to listen to my own heart, in more ways than one. I never examined how my story as a White man who grew up in rural Minnesota as the descendant of German, Irish, and French settlers might affect patient care. I never considered how the illness narratives of my upbringing or the culture of biomedicine shaped my assumptions about health. With my stethoscope's bell continuously pointed away from myself on a longer and longer tether, I became skilled at maintaining distance from the social context of both myself and my patients.

Inventing Distance

René Laënnec, a White European, invented the stethoscope in 1816 not because it generated clearer heart sounds but because it eased the discomfort of gendered social norms. Clinicians at that time sought freedom from the muddying interference of social context, economic pressures, and political ideology to purify objective recognition of

disease.¹ Stethoscopes supported these efforts by amplifying bodily sounds over the distracting background of **patient voice**.

Today, as a primary care physician, I continue clinging tightly to my stethoscope while participating in systems that perpetuate distance. Hurried encounters force us clinicians to interrupt patients. Electronic medical records dominate our attention and distract from human interaction. Telehealth eliminates physical presence, which risks creating isolation and disconnectedness. Billing tallies the use of the stethoscope and other distancing tools, not whether a clinician listened well to human stories.

Consequences of Distance

Our continuous pursuit of distance costs us clinicians a great deal. Meaningful opportunities for human connection are lost. During a recent clinic visit with me, a patient who moved to Minnesota from Vietnam in the 1980s started weeping about her husband's death from pulmonary fibrosis. She quickly stopped, however, wiped her tears, and said, "I'm sorry I'm wasting your time." Thirty years in our health system had taught her that clinicians considered her emotional and relational life to be a waste of their time.

With distance, we blame patients for their conditions instead of seeing the oppressive social forces that sicken humans. I recently spent an entire clinic visit preaching antiviral medication adherence to a 50-year-old male born in Myanmar with a rising hepatitis B viral load. Only later did I learn that high-deductible insurance paired with a low-paying job made purchase of the medication impossible for him.

We avoid examining our complicity in reproducing harmful social forces. We miss how we uphold White supremacy through race-based adjustments of estimated glomerular filtration rate and spirometers.² We ignore our control and marginalization of people experiencing homelessness, imprisonment, and forced migration.³ Honestly mirroring the **ableism of our systems**, a middle-aged female born in Myanmar recently asked me in clinic, "Am I normal?" Puzzled, I asked for clarification, and she responded, "Every time I come in you tell me something is wrong with me."

Indeed, the clinic walls and our hearts are not impermeable to social context. Since the stethoscope's invention, racism, racial capitalism, patriarchy, and ableism have both originated within and penetrated the clinic. Instead of confronting this history head-on, we continuously lengthen the tubing of our stethoscopes, thereby disconnecting ourselves from patients and undermining our desire to reduce suffering and heal.

Pursuing Proximity

How and where might we look in order to reverse course? To start, we could make the radical about-face of turning our stethoscopes upon ourselves. Somatic therapists urge connection first with our own bodies in order to begin healing disconnection and dehumanization.⁴ Integrating embodied practices, such as palpating our own pulsating hearts or tightened abdominal walls as we provide clinical care, generates stamina for honestly grappling with the privileges, biases, trauma, and strengths in our own stories.

With settled bodies, we are then ready for transformational connection with patients and communities. We practice narrative health. We ask about and affirm patients' resilience and strength. We learn history from the perspective of Black, Indigenous, and other historically oppressed communities. With those voices in mind, we walk around our own

neighborhoods and those of our patients. We do home visits. We coordinate care with community organizations working on affordable housing, food security, and immigration. We learn from and refer to other healing traditions. We seek out community artists who reveal insights that no randomized control trials ever can. We accompany community health workers. We learn from nurses who organize collectives. We engage with and keep engaging with racial justice training. We take a social medicine course and then teach one. We communicate with and testify in front of elected officials. We work to integrate health care into emerging solidarity economies.

Incorporating these ideas into health work is not new. In the United States, somatic therapists are actively creating a global network to heal embodied trauma.⁵ In Cuba, medical students visit thousands of homes to prepare for careers living and working in the same neighborhoods as their patients.⁶ In Rwanda, physicians support a national network of community health workers who provide frontline care.⁷ Clearly, we are not short on the imagination needed for pragmatic and visionary solutions. What we have lacked is the courage and the will to collectively turn around on the well-worn path of distance that medicine has traveled.

A Portal

Amidst the disruptive twinning of the COVID-19 pandemic and protests **demanding racial justice**, we stand facing what has aptly been described by Arundhati Roy as a portal.⁸ In health care, the terrain at our portal's entrance holds tremendous contradiction. We hear both the eerie hush of community clinics quieted by decreased patient visits and staff furloughs and the clamor of frenzied intensive care units operating near capacity. We hear health system leaders denouncing racism while workers and patients in those systems decry racial hierarchy in day-to-day practices. We witness people experiencing homelessness being evicted from encampments while public health officials preach sheltering at home and social distancing to prevent COVID-19 infection.

Passage into a portal offers the chance to strengthen the pulsations of reimagining and re-creation. In this moment, we can listen more clearly and more closely both to ourselves and to social context. When we do so, it becomes evident that distance from social context doesn't free us from harmful social systems and structures—proximity does. We will see that if we desire to care with compassion, heal holistically, and advance health equity, we must at times set down the stethoscope.

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