

CASE AND COMMENTARY: PEER-REVIEWED ARTICLE

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

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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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Editor's Note

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