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Abstract

The second section of our Special Task Force builds on the discussion of value and perspective in the previous article of the report by 1) defining a health economics approach to the concept of value in health care systems; 2) discussing the relationship of value to perspective and decision context, that is, how recently proposed value frameworks vary by the types of decisions being made and by the stakeholders involved; 3) describing the patient perspective on value because the patient is a key stakeholder, but one also wearing the hat of a health insurance purchaser; and 4) discussing how value is relevant in the market-based US system of mixed private and public insurance, and differs from its use in single-payer systems. The five recent value frameworks that motivated this report vary in the types of decisions they intend to inform, ranging from coverage, access, and pricing decisions to those defining appropriate clinical pathways and to supporting provider-clinician shared decision making. Each of these value frameworks must be evaluated in its own decision context for its own objectives. Existing guidelines for cost-effectiveness analysis emphasize the importance of clearly specifying the perspective from which the analysis is undertaken. Relevant perspectives may include, among others, 1) the health plan enrollee, 2) the patient, 3) the health plan manager, 4) the provider, 5) the technology manufacturer, 6) the specialty society, 7) government regulator, or 8) society as a whole. A valid and informative cost-effectiveness analysis could be conducted from the perspective of any of these stakeholders, depending on the decision context. Keywords: cost-effectiveness analysis, decision context, perspective, value.

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Introduction

This section builds on the discussion of value and perspective in the introduction by Neumann et al. [1] by 1) defining a health economics approach to the concept of value in health care systems; 2) discussing the relationship of value to perspective and how recently proposed value frameworks vary by the types of decisions being made—what we call “decision context”—and by the stakeholders involved in those decisions; 3) describing the patient perspective on value because the patient is a key stakeholder, but one also wearing the hat of a health insurance purchaser; and 4) discussing how value is relevant in the market-based US system of mixed private and public insurance, and differs from its use in single-payer systems.

Defining Value

Given the focus of our Special Task Force (STF) on US value assessment frameworks that exist in a mixed private and public health insurance system with a market orientation, our approach follows basic accepted principles of microeconomics, even as we recognize 1) certain limitations of these principles, particularly in relation to health care markets, and 2) the need to consider important issues, such as equity, that are not traditionally the focus of economics and are addressed by other disciplines. First, “value” is defined here from an economic perspective: the “gross value” can be thought of what someone would be willing to pay for an economic good or intervention, whereas the “net value” subtracts the opportunity cost incurred to obtain that gross value.

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In other words, the net value is what a consumer would be willing to pay to avoid losing access to the good. Second, because individuals vary in their preferences for health and other economic goods, the value they place on different health care interventions will also vary. Third, given that most medical care is purchased indirectly via health insurance, individuals do not directly face prices, and their agents (insurers and providers) acting on their behalf must assess value for money. Indeed, this is a large part of the motivation for payers and providers to develop value frameworks. Sometimes US payers have established price schedules (e.g., Medicare physician fee schedules or prospective payment by diagnosis-related groups), although their link to value is arguably tenuous.

The concepts of value and efficiency are related in economics, but the relationship can be complicated and nuanced with respect to health care technologies—and notably for innovative medicines. For economic assessment purposes, the net value of an action, a program, a treatment, or a technology reflects the willingness to pay (WTP) for the improvement in well-being minus the opportunity cost of resources used to produce that improvement. Broadly, achieving “economic efficiency” is obtaining maximum value for the money spent. In assessing value and economic efficiency from the perspective of society as a whole, both the well-being (utility) and the cost measures should include the consequences to all those affected by the action [2].

For well-known reasons, directly assessing benefits and costs of health care in strictly monetary terms using observed market prices is fraught with difficulty given the market distortions (such as insurance), as noted earlier. Cost-effectiveness analysis (CEA), by relating an intervention’s cost to its effectiveness (in terms of some change in health) as a ratio, is thus a standard approach to measuring the net value of a health care intervention. Economic efficiency is also sometimes considered in the short-term (static efficiency) and in the long-term (dynamic efficiency). Static efficiency means, for example, achieving the maximum expected health gain from a fixed annual budget; dynamic efficiency is about achieving the optimal rate of innovation. They are related in that paying for what people value provides the incentive to direct research and development to produce innovations that generate that value [3]. Because a simple comparison of market-based monetary benefits and costs is not feasible or useful for most individual health care technologies, health economists and outcomes researchers have developed a work-around that allows comparisons among technologies in terms of incremental cost-effectiveness ratios, and in comparison with a threshold of WTP or opportunity cost [4–6].

Empirical studies document what is known intuitively about heterogeneous preferences for health care. Our fellow citizens attach different values to health outcomes. Two patients might value the same health gain differently, and some of this variation is related to differences in incomes, but much of it is related to differences in preferences about spending on the health care versus other non–health-related goods that they value. There is also variation across people in the extent to which they are willing to trade off extensions in life expectancy against various aspects of the quality of life. Nevertheless, the larger concern in heterogeneity is the health gain delivered from the same intervention in different subpopulations. This heterogeneity of treatment effect implies that it is inappropriate to try to determine a single value for a medical intervention; rather, we should seek to know the distribution of values in a population. Payment and reward systems for medical products and services are, however, typically based on population averages, and hence cannot fully reflect this variation in value.

The theoretical foundations chapter of the recently published report [2] of the Second Panel on Cost-Effectiveness in Health and Medicine posits that alternative microeconomic approaches are variants of the general principle of “constrained optimization,” in particular, the maximization of health (a form of extra-welfarism) [7] or the maximization of individual well-being (traditional welfare economics) subject to a budget constraint [4]. Our STF follows a welfare economics approach in considering US value frameworks, recognizing that there is no fixed annual global budget for all of health care in the United States. At the same time, there are clearly more specific resource constraints and opportunity costs that apply over time in obtaining care for health plan members. Moreover, in public programs such as Medicaid or for public payers with short-term time horizons, there may effectively be a fixed annual budget.

Stakeholders, Perspectives, and Decision Contexts

Numerous guidelines for CEA emphasize the importance of clearly specifying from whose perspective the analysis is being undertaken [2,8]. In other words, from the standpoint of which key decision maker is the constrained optimization being undertaken: 1) the typical health plan enrollee, 2) the patient, 3) the health plan manager, 4) the provider, 5) the technology manufacturer, 6) the specialty society, 7) the payer (public or private), or 8) society as a whole. A valid and informative CEA could be conducted from the perspective of any of these stakeholders, depending on the purpose of the analysis.

Most published CEAs in the United States aim to support public or private health sector decision making about what technologies or strategies should be available to health plan members when those members become patients with a specific illness or condition.

The report of the Second Panel [2] is instructive in that the authors re-examined the past 20 years of the practice of CEA since the publication of the original panel’s report. They identified and defined four normative perspectives for consideration, ranging from the least to the most generally inclusive:

1. payer perspective;
2. health care sector perspective;
3. health care sector with time cost perspective; and
4. societal perspective.

Although any of these (as well as other possible perspectives) can be taken in CEAs and be scientifically valid and informative for certain decision makers, the Second Panel recommended—for the sake of consistency and comparability—that analysts should report “reference cases” from two perspectives—the health care sector perspective and the societal perspective.

The health care sector perspective includes “formal health care sector (medical) costs borne by third-party payers or paid for out-of-pocket by patients.” This includes “current and future health costs, related and unrelated to the condition under consideration [2].” Notably, it does not include patient time costs or the future benefits and costs of other types of consumption associated with increased longevity. In contrast, the more narrowly construed “payer perspective” does not include patient out-of-pocket costs because they are not borne by payers.

The Second Panel’s recommended societal perspective is very broad, adding time costs and effects on future productivity and consumption as well as relevant non–health-related impacts in other sectors, such as education and criminal justice. The panel recommended an “Impact Inventory” (reproduced here in Fig. 1), a structured table or checklist listing an intervention’s health-related and non–health-related effects, as a way of communicating to audiences the components included in a CEA and whether and how they were valued [9].
As shown in Figure 2, the five recently proposed US value frameworks reviewed here, as identified in the article by Neumann et al. [1], have similarly tended to take a normative view, but have taken varying perspectives and have addressed varying decision contexts. As illustrated at the bottom of Figure 2, for example, the American Society of Clinical Oncology [10] and the National Comprehensive Cancer Network [11] frameworks operate in the context of what is often called “clinical shared decision making,” in which oncologists, as agents of patients, work with patients to help maximize each patient’s health and well-being. In contrast, at the top level in the figure, the Institute for Clinical and Economic Research framework aims to inform the payer’s decisions about which products to include in a formulary and what constitutes a “value-based” price. The American College of Cardiology/American Heart Association [12] recommendations pertain to decisions about how to construct clinical guidelines or pathways in treatments or prevention in cardiac care, which would presumably guide, influence, or constrain the options considered under clinician-patient shared decision making. The Memorial Sloan Kettering Cancer Center DrugAbacus [13] addresses both the payer and the patient decision contexts with an emphasis on oncology drug prices. As in the 1997 seminal work by Garber and Phelps [4], this STF focuses on optimizing value from the standpoint of an individual seeking to purchase the right to obtain a new medicine or other medical technology under an actuarially fair insurance plan in a market with competing health plans; this can affect both what is in the plan and the total amount of insurance purchased. And similar to the Second Panel, this orientation leads to consideration of both the health care sector perspective, where the health plan can be thought of as the agent of the plan member (and the patient), and the societal perspective, which considers both the health-related and the non-health-related implications for all stakeholders.

One might ask what person or institution actually takes a societal perspective (or the “social welfare perspective” as it is called in traditional public finance economics). That perspective aims to provide a complete description of costs and benefits regardless of who bears them. This could be thought of as the broadest “public interest” decision context, which is that of an
Population-level decision contexts (i.e., decisions made under a health plan, health care sector, or societal perspective) for individual treatments can recognize individual patient preferences and their diversity, in particular ways. In the article by Lakdawalla et al. [14], we review elements for capturing aspects of patient preferences, or utilities, which can help population-level decisions be more patient-centric. In reality, CEA is typically performed using population averages with only limited flexibility to account for patient heterogeneity. Because sources of patient heterogeneity such as baseline risks, age, or disease severity can be observed, they can be used in utilization management. Other sources of patient heterogeneity, for example, difference in treatment effects, patient health state preferences, or patient WTP, may not be easily or objectively observed, especially outside the patient-provider decision context. Generally, however, population averages are used in CEA only in terms of preferences. If a given treatment is marginally cost-effective overall, but has higher value to a particular patient for less observable reasons, a possible strategy would be for plans to allow the patient access but require that they pay a higher co-pay or fulfill a step therapy or prior authorization criterion. Payers may have to estimate how much of a margin above a given population cost-effectiveness threshold or opportunity cost will allow appropriate access to a treatment for patients who value it more highly than average cost effectiveness would indicate; patient advocacy for a treatment at the plan level can provide signals that influence those margins.

It is rational for patients to choose among the treatments available to them as per the conditions of their insurance plans, on the basis of their own health status, preferences, and incentives (e.g., out-of-pocket costs). This choice would not reflect population-level cost effectiveness but rather individualized value expectations based on a thorough and reasoned comparison with their treatment options. In reality, however, there are limitations on the information that patients typically have about treatment options and their effectiveness, side effects, costs, and possible outcomes; on their ability to deal with probabilities; and other aspects of valuation and decision making (sometimes called “bounded rationality” [15]) as well as cognitive and other biases that affect their decision making [16]. In addition, the treating physician or other parties may provide information (and introduce their own biases) and influence a patient’s decision in a way that may not be entirely based on the patient’s own values. Technically, this is called the “principal-agent problem” [17,18].

Thus, in addition to the limitations of insurance coverage, the values and incentives of other parties, especially the provider, may affect treatment choice. Despite these complexities, a patient-centric approach means that the patient’s perspective remains largely unchanged regardless of whether value expectations based on a thorough and reasoned comparison with their treatment options. In reality, however, there are limitations on the information that patients typically have about treatment options and their effectiveness, side effects, costs, and possible outcomes; on their ability to deal with probabilities; and other aspects of valuation and decision making (sometimes called “bounded rationality” [15]) as well as cognitive and other biases that affect their decision making [16]. In addition, the treating physician or other parties may provide information (and introduce their own biases) and influence a patient’s decision in a way that may not be entirely based on the patient’s own values. Technically, this is called the “principal-agent problem” [17,18].

Thus, in addition to the limitations of insurance coverage, the values and incentives of other parties, especially the provider, may affect treatment choice. Despite these complexities, a patient-centric approach means that the patient’s perspective remains largely unchanged regardless of whether value
is being measured within a market-based or a single-payer health care system. Researchers generally measure value using the tool of CEA. Nevertheless, how these measures are used for decision making has some important similarities and differences in market-based health care systems versus single-payer systems such as the UK National Health Service (NHS). In a general sense, the difference stems from the emphasis in market systems on “consumer sovereignty” versus “social solidarity” in single-payer systems.

In one example of a single-payer system—that in the United Kingdom—the emphasis is on all citizens having equal access to the same health benefit package. CEA is used not only to evaluate what goes in the package that aims to be available to everyone, but also as a tool to negotiate prices with manufacturers. Indeed, given the existence of a social WTP threshold, it was recognized several years ago by the Office of Fair Trading [20] in the United Kingdom that this could provide the basis for a ‘value-based pricing’ system for new medicines. Nevertheless, the effort to implement such a system with a broader concept of value failed [21]. There was a return to the use of the cost-per-quality-adjusted life-year (QALY) ratio in relation to a WTP threshold. Nevertheless, although controversy continues regarding the appropriate level of threshold, the NHS has increasingly engaged in confidential discounts with manufacturers that would seem to approximate value-based prices at the national level. For the most part, however, individual UK citizens do not have the opportunity to pay out of pocket for additional services based on their personal valuations of health technologies.

In a market-based system, however, decision making is decentralized. Thus, consumers making decisions about the purchase of private health insurance or out-of-pocket spending may vary in their objectives and preferences. For instance, they may differ greatly in the value they attach to increments to health and/or the contribution of the new technology to their health. Some may expect relatively modest benefits to which they attach modest value per unit of health, whereas others may receive life-saving benefits and attach great value to additional life-years and quality of life. In this situation, there can be no single correct or unique value-based price for all patients or plan enrollees; instead, it would vary across members of the population. Any effort to establish a single measure of value or a single value-based price ignores the variations in individual preferences and thus may lead to inefficient resource allocation both within the health system and in non-health-related spending [22].

<table>
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<th>Table 1 – Key stakeholders in different decision contexts.</th>
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<td>Decision context</td>
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<td>Health plan enrollment</td>
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<td>Health plan coverage and reimbursement</td>
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<td>Practice guideline development</td>
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<td>Clinical shared decision making</td>
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Note. ‘X’ is a key stakeholder in a given decision context. ACC, American College of Cardiology; AHA, American Heart Association; CEA, cost-effectiveness analysis.
In a market-oriented system such as in the United States, one possible scenario is that health plan enrollees could choose among competing managed care insurers on the basis of the value the enrollees attach to additional health and their net benefit from the new technology. In this sorting process, each insurer could describe the maximum price they would be willing to pay (e.g., for QALY gains) on behalf of its members, and then see how many members it attracts. This variation in value and effectiveness would trace out the demand curve, which represents the distribution of value-based plan premiums in the population. Whatever price is set for a given health plan, all potential enrollees with values greater than or equal to that price will purchase the policy. This would be the value-based premium for them. Those who have lower values will “walk away” (i.e., will choose the “exit” option) because their value-based premium is lower than the price the plan chose to set.

One interesting similarity in the United Kingdom and the United States in the decision context of clinical treatment guidelines is that the American College of Cardiology/American Heart Association Joint Task Force has recently recommended and taken steps to use CEA and thresholds in defining appropriate clinical pathways [12]. CEA has been applied in the UK NHS in developing treatment guidelines by disease area, although the paucity of CEA data can make it difficult to do [23].

The US health insurance system is, of course, not entirely market-based and hence is pluralistic. The Medicaid and Medicare programs, which are largely publicly funded, reflect this. The task of specifying a value-based price can be different for these and other government programs than for private insurers. For Medicaid, one can hypothesize that there is some consensus value of increments to health among taxpayers who support the program, and that value multiplied by the increment in health yields a value-based price that the seller may or may not choose to accept. The WTP of beneficiaries is so small enough relative to cost that it can usually be ignored. The political rules for combining disparate voter preferences are varied. No one rule is ideal, and so there is some ambiguity.

For Medicare, the question is whether the WTP of beneficiaries, which can be substantial, should play a role along with that of their children and grandchildren who will be bearing the bulk of the financial burden. Given Medicare’s prohibition on balance billing, those beneficiaries with high values will not be able to express such values, and given Medicare’s low cost-sharing (made even lower by Medigap policies), the WTP for cost-sharing by beneficiaries does not furnish a reliable guide to determine a price reflecting net value.

Nonetheless, even though the US health care system is not a full and free market system, it remains difficult to argue that a single assessment of value will work well for the full range of relevant decision makers. For this reason, US value assessments must grapple with diversity in preferences and in the willingness and ability to pay.

**Conclusions**

To reiterate and underscore some key points in this section of the report, “value” is defined here from an economic perspective, considering both the gross value—what someone would be willing to pay for access to and use of a health care technology—and its net value, taking account of “opportunity cost”—what they have to give up to get it. The five recent value frameworks that motivated this STF vary in the decisions that are being informed by the valuation, ranging from coverage, access, and pricing to defining appropriate clinical pathways and to supporting clinician-patient shared decision making. Each of these value frameworks must be evaluated in its own decision context for its own objectives. Existing guidelines for CEA emphasize the importance of clearly specifying the perspective from which the analysis is undertaken. Relevant perspectives may include, among others, 1) the typical health plan enrollee, 2) the patient, 3) the health plan manager, 4) the provider, 5) the technology manufacturer, 6) the specialty society, 7) government regulators, or 8) society as a whole. A valid and informative CEA could be conducted from the perspective of any of these stakeholders, depending on the purpose of the analysis. This economic concept of value does not depend on whether value is being measured within a market-based or a single-payer health care system. Health economics and outcomes researchers generally measure value using the tool of CEA. CEA is patient-centric in building up the valuation from the impacts on patient length and quality of life. In a market-based system, the individual—wearing the two hats of the plan subscriber and potential patient—is the ultimate decision maker, making these decisions with the assistance of agents—the insurer and providers. Consumers making decisions about the purchase of private health insurance or out-of-pocket spending may vary in their objectives and preferences. As a result, they will choose different health plans that have different WTP for QALYs and so different cost-effectiveness thresholds.

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**References**


