Toward a Broader Concept of Value: Identifying and Defining Elements for an Expanded Cost-Effectiveness Analysis

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A B S T R A C T

This commentary identifies and defines potentially useful expansions to traditional cost-effectiveness analysis as often used in health technology assessment. Since the seminal 1977 article by Weinstein and Stason, the recommended approach has been the use of the incremental cost-effectiveness ratio based on the metric of the cost per quality-adjusted life-year gained, allowing comparisons across different technologies. An expanded framework, incorporating a wider range of the elements of value, is proposed. In addition to the core value drivers of health gain and other health system cost savings (if any), we propose adding other less recognized elements related to the value of knowing and informational externalities. We describe each of five factors related to the value of knowing: 1) a reduction in uncertainty, reflecting the benefit of a companion diagnostic increasing the certainty of a patient’s response to a medicine; 2) insurance value related to greater peace of mind due to protection against catastrophic health and financial loss; 3) the value of hope for a “cure,” leading individuals to become risk seekers in some circumstances; 4) real option value due to life extension opening possibilities for individuals to benefit from future innovation; and 5) spillovers or externalities arising from benefits of scientific advances that cannot be entirely appropriated by those making the advances. Further thought and research are needed on how best to measure and integrate these elements into an incremental value framework and on coverage and pricing decisions.

Keywords: cost, cost-effectiveness analysis, value frameworks, value of knowing.

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Introduction

In the last 2 years, several health care “value frameworks” have been promulgated in the United States [1]. Motivations for these frameworks vary, but their emergence in part reflects increased pressure to constrain US health care spending, particularly in oncology. New hepatitis C treatments have also raised concerns affecting the value of health care interventions, many have argued that patients value other features of health care.

Of course, “value” in relation to health means different things to different individuals. In most instances, ill patients first want improved health—in terms of improved survival or quality of life or both. Additionally, from the health sector point of view, any reduction in the resource costs of treating illness is also valuable. The simplest definitions of value tend to cite a ratio or relationship between costs and health outcomes (e.g., “value is defined as outcomes relative to costs” [2]). This ratio is a version of the widely used incremental cost-effectiveness ratio (ICER), the gold standard for economic evaluation of health care technologies. The Tufts Cost-Effectiveness Analysis (CEA) Registry has published estimates for over 3700 ICERs [3].

Almost 40 years ago, Weinstein and Stason [4] provided the foundation for CEA in health care by defining and recommending the use of the ICER, using the metric of cost per quality-adjusted life-year (QALY) gained, as a proxy for improvements in health, thereby allowing comparisons across technologies. Although health gains (improving the well-being of patients) and cost offsets (which are usually only partial) are the core value drivers of health care interventions, many have argued that patients value other features of health care.

In this commentary, we identify and describe a specific subset of these additional elements of value that affect consumer welfare in relation to aspects of mental well-being—for example, related to “peace of mind.” These have previously been identified, if not fully developed in the literature; however, little attention has been given to representing them in a more comprehensive,
expanded CEA framework for assessing value. This commentary represents a step toward addressing this gap.

Need for an Expanded CEA Framework

We believe that recently proposed value frameworks have significant shortcomings in concept and measurement. Some of their relative strengths and weaknesses for their stated purposes have been noted [1]. An expanded framework that incorporates a wider range of the elements of value is needed, in part to reflect the growing importance of interventions that use complementary diagnostics to target medicines via “precision medicine”; offer “cures”; or provide access to future innovation by extending life [5].

In addition to the “core” elements of value of health gain (usually measured in QALYs) and any health system cost savings, we propose that other elements also deserve consideration. We would include commonly recognized elements (e.g., productivity and impacts on other economic sectors) but also less frequently cited factors related to information and different aspects of value related to knowing. These information-related factors can reduce uncertainty and thus improve mental well-being and simultaneously create more value in the aggregate through expanded, appropriate use in a population. This article defines some of the less recognized elements.

From an economic perspective, the concept of value is reflected in the demand curve that describes consumers’ willingness to pay (WTP) for different quantities demanded at varying price levels. Estimating this relationship is difficult in health care given that consumers make these purchases indirectly through the veil of private or social insurance. For the sake of this discussion, we refer to this WTP as “economic value.” The plan member relies on the health plan to make an appropriate value assessment in its coverage decision. The maximum price the plan should pay for a technology is equal to this economic value. As we noted earlier, the core elements of value are health gain and any offsetting cost savings that result from use of the technology.

Expanding the Elements in CEA

Twenty years after Weinstein and Stason [4], Garber and Phelps [6] derived the cost-per-QALY threshold within a welfare economics framework, that is, from a utility-maximizing consumer of health care. Individuals have different incomes and preferences for health and will have different cost-effectiveness thresholds. They can use policy coverage “choice” in market-based systems and “voice” in social insurance/tax-based systems to meet their health, budget, and risk preferences, including preferences for health for their fellow citizens [7].

The intellectual underpinning of this “welfarist” approach differs from an “extra-welfarist” approach focusing on supporting a collective decision maker in maximizing health gains given a budget or other constraints [8,9], which implies a more straightforward cost-effectiveness threshold. Both approaches can include factors other than health gain and cost savings (e.g., equity or “wider societal benefits”), although formal incorporation into decision making can be difficult [10]. Recognition that consumer welfare includes aspects of mental well-being, such as peace of mind or reassurance about protection, opens potential additional elements of value related to risk protection as well as information that can increase choice or provide reassurance. These are most readily conceptualized in a welfarist framework but could also be valued and included within an extra-welfarist decision-making framework.

Five elements related to the “value of knowing” (distinguished from the “value of information”) have been summarized in a recent white paper on personalized medicine [5]. We would argue, however, that they have broad applicability to the full range of health technologies. The first three relate to patient and plan member attitudes to risk, which, as well as having reassurance value, can lead to different treatment decisions. Indeed, only the first of these three—reduction in uncertainty for the patient—is related specifically to personalized medicine. The fourth relates to the value of an option created by treatment. The fifth relates more generally to the knowledge externalities created by new treatments. Figure 1 illustrates these additional elements in relation to the more customary ones. The five are briefly described here:

- **Reduction in uncertainty** arising from the use of a diagnostic test. There has been a growth in new medicines with complementary diagnostics; these medicines avoid adverse events and waste by concentrating treatment on patients who can benefit most. By increasing the certainty of a patient’s response to a medicine, value is created for the patient over and above the expected health gain. Furthermore, patients would be willing to pay more for the combination. At a population level, greater certainty could lead to greater uptake and improved compliance [11–13]. This effect arises from the attitude of the patient to the risk associated with treatment. For a risk-averse patient, reduced variance around the expected health outcome increases the value of the health outcome. This effect differs from the benefits of a value-of-information calculation to a risk-neutral payer, which may identify additional value from additional research [14]. There is a value of knowing even in extreme circumstances when no treatment is available. Neumann et al. [15] found WTP for a test in these circumstances. The rationale is not difficult to find: A test would enable people to make decisions about how to spend the remainder of their lives.

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**Fig. 1 – Elements of Value.**

Notes: Dark blue circles: proposed information-related elements of value. Light blue circles: traditional HTA and other societal elements of value. Blue line: value element in traditional HTA/health system perspective. Red line: additional value element also included in societal perspective. Source: Adapted from Figure 2 in Garrison et al. [5].
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