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Evaluating Frameworks That Provide Value Measures for Health Care Interventions

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ABSTRACT

The recent acceleration of scientific discovery has led to greater choices in health care. New technologies, diagnostic tests, and pharmaceuticals have widely varying impact on patients and populations in terms of benefits, toxicities, and costs, stimulating a resurgence of interest in the creation of frameworks intended to measure value in health. Many of these are offered by providers and/or advocacy organizations with expertise and interest in specific diseases (e.g., cancer and heart disease). To help assess the utility of and the potential biases embedded in these frameworks, we created an evaluation taxonomy with seven basic components: 1) define the purpose; 2) detail the conceptual approach, including perspectives, methods for obtaining preferences of decision makers (e.g., patients), and ability to incorporate multiple dimensions of value; 3) discuss inclusions and exclusions of elements included in the framework, and whether the framework assumes clinical intervention or offers alternatives such as palliative care or watchful waiting; 4) evaluate data sources and their scientific validity; 5) assess the intervention's effect on total costs of treating a defined population; 6) analyze how uncertainty is incorporated; and 7) illuminate possible conflicts of interest among those creating the framework. We apply the taxonomy to four representative value frameworks recently published by professional organizations focused on treatment of cancer and heart disease and on vaccine use. We conclude that each of these efforts has strengths and weaknesses when evaluated using our taxonomy, and suggest pathways to enhance the utility of value-assessing frameworks for policy and clinical decision making.

Keywords: cost-effectiveness, multi-attribute decision analysis, value frameworks.

Introduction

The explosion of scientific knowledge, discovery, and technology began a new era of health care, wherein deployment of health care interventions depends on personal, lifestyle, genetic, and molecular disease factors. Although these advances have the potential to improve health, they increase the complexity of analyses to determine value at reasonable and affordable costs [1–8].

Faced with new health care interventions and escalating costs of care, many professional organizations have developed frameworks to measure and/or rank the value of novel interventions [3,7,9–22]. Some of these new frameworks use components of cost-effectiveness analysis (CEA), whereas others create de novo value measures. It remains unclear how such frameworks will guide clinical practice policy, and whether they offer particular advantages over traditional approaches such as CEA.

First, we summarize a taxonomy of principles—expanding upon cost-effectiveness theory—that provides an approach to assess different value frameworks. Next, we apply this taxonomy to evaluate exemplar value frameworks to illustrate strengths, gaps, and potential concerns. We intend for this synthesis to stimulate discussion about best practices and use of value metrics in policy and clinical decisions. We derived these principles in our synthesis of standard practice for the reporting of CEAs [23–25], recent International Society for Pharmacoeconomics and Outcomes Research summaries of multicriteria decision analysis (MCDA) reporting guidelines [26], standard medical journal requirements for conflict of interest disclosures [27], standard Cochrane review methods [28], and our own analysis. Although we draw many of these ideas from others, assembling them to evaluate models that estimate the value of health care is novel.

Value Taxonomy

Value frameworks can assist in public health prioritization, practice guidelines, formulary or related resource expenditure decisions (e.g., purchase of equipment or coverage of a specific

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Table 1 – Proposed taxonomy for evaluation of frameworks assessing the value of health care interventions.

Category	Component details
Purpose	Is the purpose defined and are the elements of the framework consistent with the purpose?
Conceptual approach, perspective, and preferences	Is the value structure of the framework clear and transparent? If it requires assumptions, do they have general and clinical face validity? What perspective does the framework use (e.g., societal, patient, provider, payer)? Is the perspective made explicit or embedded in the framework? Does the framework include standard methods of measuring value, such as cost-effectiveness analysis? Does the framework consider multiple attributes of medical interventions such as individual preferences, equity and the distribution of health care benefits and costs, issues involving externalities (such as contagious diseases), and the value of scientific breakthrough? If the framework includes multiple dimensions of value, are the weights for each component population-based or expert-provided, and are the methods for eliciting such weights both clearly stated and methodologically sound? Does the framework allow individual patient preferences? If so, are they elicited with methods known to be free of bias and to produce reliable results?
Intervention components and comparators	Does the framework include all components and consequences of the intervention, or merely a portion of those (e.g., drug acquisition costs)? Does the framework aggregate or disaggregate such things as toxicity or other side effects of intervention? Does the framework assume as a baseline that some intervention will be provided, or does it allow for “watchful waiting” or “palliative care” as an option?
Data sources	Are clinical and other data derived from expert opinion, population surveys, or other sources? Are the sources made clear, and is the process replicable?
Economics/costs	What is the effect of the intervention on the total cost of treating a defined population, including whether inclusion of the intervention will increase, decrease, or leave unchanged the total costs of care for that population?
Uncertainty and identification of important gaps	Is uncertainty in data about costs or effects considered in conclusions about value? Are there gaps in knowledge about aspects of care that could change the value rating?
Conflicts of interest	Does the sponsoring organization have a financial stake in the process, and if so, is this declared? Does the framework bias the results in favor of the sponsor’s financial position?

drug), and patient-physician decision making about a specific care choice. Table 1 describes our taxonomy to evaluate these value frameworks. We intend for this to apply to these different purposes, but specific elements may be more or less salient for different uses of the value framework. The following section highlights the implications of these varying purposes when using this taxonomy to evaluate value frameworks.

Conceptual Approach, Perspective, and Preferences in Measuring Value

Value frameworks have evolved, in part, to provide greater standardization and transparency in health care decision making [29]. Furthermore, formal frameworks can minimize biases of intuitive decision making from issues of framing of the problem, risk analysis, intertemporal trade-offs, and others [30].

We began our taxonomy in the formal framework of CEA. CEAs most commonly use the societal perspective, but this perspective usually does not correspond to that held by individual participants in health care decisions—patients, providers, payers, public policy decision makers, or owners of resources used to create health care interventions—nor does it consider community well-being or other important policy issues such as the distribution of benefits and costs across various subsets of the population (among other omissions).

No single perspective can represent the interests of all participants in value-based decisions. For example, physicians generally advise patients using a patient perspective, but may introduce the societal perspective in their role as the steward of societal resources [31] or, in some cases, their own professional organization’s or personal financial situation. Thus, we believe that multiple perspectives should be considered, varying by individual or organization assessing the intervention, consistent with the most recent recommendations for reporting CEAs [24,25].

CEA can also be useful in developing value frameworks because it provides data on intermediate events of importance to judging the value of an intervention, such as toxicity rates and overall survival, measured in life-years saved, quality-adjusted life-years, or disability-adjusted life-years [23–25,32,33]. Compared with the existing care standard, the ratio of added costs to added gains in health benefits—the incremental cost-effectiveness ratio—stands as one value criterion. Nevertheless, no consensus exists on cutoff points to determine the “acceptability” of a given incremental cost-effectiveness ratio, and both illness severity and the number of people affected can influence “cutoff point” choices.

CEA also does not typically capture less tangible factors such as the fear associated with diseases such as HIV, cancer, degenerative neurological conditions, or mental illness, because these do not represent distinct health outcome states for which utility measurements exist. In concept, CEA could incorporate such issues, but in many cases, the data requirements would be impossibly complex. Full CEA evaluation requires population-based utility-level estimates for every state of nature in the model. Capturing such things as fear of the diseases, contagion effects, restrictions on freedom, and modification of religious rites (all arising in response to the Ebola virus) provides examples of issues that CEA cannot meaningfully capture.

Although grounded in CEA, our taxonomy extends beyond this foundation. CEA does not generally consider many other attributes of decision making that affect the use of health care interventions. For example, CEA cannot address the