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Review of Recent US Value Frameworks—A Health Economics Approach: An ISPOR Special Task Force Report [6]

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ABSTRACT

The sixth section of our Special Task Force (STF) report reviews and comments on recent US-oriented value assessment frameworks, specifically those published by the American College of Cardiology/American Heart Association, the Institute for Clinical and Economic Research, the American Society of Clinical Oncology, the National Comprehensive Cancer Network, and the Memorial Sloan Kettering Cancer Center. We review published commentaries that address the validity, reliability, and conceptual underpinnings of these frameworks. We find common themes of critique regarding the strengths and limitations across frameworks. Particular shortcomings of some frameworks pose greater threats to their face validity and utility compared with others. The most significant limitations include lack of clear perspective (e.g., patient vs. health plan) and poor transparency in accounting for costs and benefits. We then review how each framework adheres to core STF recommendations, with particular emphasis on whether the framework can be used to support coverage decisions by health insurers, and whether it adheres to core principles of cost-effectiveness analysis. The Institute for Clinical and Economic Research framework most closely adheres to core STF recommendations. Others have significant limitations that vary widely from framework to framework. We also review how the frameworks follow STF recommendations for addressing potentially relevant issues beyond cost-effectiveness analysis - for example, equity in resource allocation and patient heterogeneity. Finally, we review whether and how each framework uses value thresholds and addresses affordability concerns. We conclude with suggestions for further research, particularly in the areas of testing the measurement and use of novel elements of value and deliberative processes.

Keywords: guiding principles, health technology assessment, methodological issues, value frameworks.

Introduction

The work of our Special Task Force (STF) complements previous critiques of US value frameworks (e.g., Refs. [1–5]). In this section, we briefly review the relevant frameworks, summarize several previous reviews, and provide suggestions for those frameworks [6–11].

Recent US Value Frameworks

The five recent US value frameworks identified and described in the articles by Neumann et al. [12] and Garrison et al. [13] of our STF report can be broadly characterized in terms of their decision contexts and perspectives, which in turn determine how each framework accounts for costs and health outcomes. The frameworks are diverse in how they define and measure value [1,12,13]. The Institute for Clinical and Economic Review (ICER) and the American College of Cardiology/American Heart Association (ACC/AHA) frameworks use conventional cost-effectiveness analysis (CEA) with quality-adjusted life-years (QALYs) to support population-level recommendations (including payer, health sector, or societal perspective). The ICER framework is oriented toward health plan coverage and reimbursement decisions, and it also applies CEA with QALYs and estimates population-level budget impact to address affordability when applicable. The ACC/AHA framework is oriented toward supporting the development of clinical guidelines and pathways. The American Society of Clinical Oncology (ASCO) and the National Comprehensive Cancer Network (NCCN) frameworks are oriented toward shared decisions by clinicians and patients for cancer drug therapies.

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As such, ASCO and NCCN eschew QALYs in favor of disaggregated measures of outcomes, drawing largely on dimensions of benefits and harms widely recognized by the oncology community. The ASCO and NCCN frameworks consider cancer drug costs only, from either the physician or the patient perspective. Memorial Sloan Kettering Cancer Center's DrugAbacus also focuses on cancer drugs and is geared toward policymakers, and allows users to specify their willingness to pay for health benefits and the importance they place on innovation and development costs. Using this information, the DrugAbacus calculator then determines the user's 'value' price for a drug indication and compares this with the list price.

Among other frameworks, the Avalere/FasterCures Patient-Perspective Value Framework (PPVF) is a "patient-centric" framework that includes a detailed set of elements that may be important to a patient's treatment decision [14]. In a somewhat similar fashion, the National Health Council's rubric sets forth a set of principles for evaluation of frameworks from a patient-centric perspective [15]. Other US-based frameworks include those recommended by the First and Second US Panels on Cost-Effectiveness in Health and Medicine as well as others listed in the article by Neumann et al. [12] of this report [11,16,17]. In Europe, many national health technology assessment bodies and the European network for Health Technology Assessment have developed value frameworks [18]. The European Society for Medical Oncology (ESMO) created a value framework on the basis of "magnitude of clinical benefit" that was similar to ASCO's, also focusing on shared decision making [19].

Given the number and diversity of these frameworks, we will not attempt a comprehensive critique. Moreover, we recognize that the frameworks continue to evolve—even during the drafting of this report, some of the frameworks have updated their approaches. This section focuses on a selected set of general concerns that are most relevant to key value and decision aspects as discussed in foregoing sections.

Critiques of the Recent US Frameworks

Conceptual Underpinnings and Methodological Issues

Some observers have criticized the new non–QALY-based frameworks for their lack of a conceptual foundation, for omitting key components of cost or outcome given their perspectives, and for not considering uncertainty. For example, Westrich [2] argues that the oncology-oriented frameworks use untested methods, are confusing in their choices for inputs and outputs, are not patient-centered, and/or do not consider system effects beyond the drug therapy. Several authors note that the ASCO framework omits several key value components, does not fully account for costs of care, and uses arbitrary methods to assign value points. The ASCO and ACC/AHA frameworks have also been criticized for not having well-articulated approaches for reflecting uncertainty [1,3,20,21].

Validity and Reliability

As with other rating scales, value assessment frameworks should demonstrate validity (the technique should measure what it intends to measure) and reliability (consistency in repeated measures by the same individual of the phenomenon under investigation) [22,23]. In a pilot study, Bentley et al. [4] found that the ASCO, ICER's evidence rating matrix, ESMO, and NCCN frameworks demonstrated convergent validity and good agreement across a sample of raters in their relative rankings of value. The ASCO and ESMO frameworks showed good inter-rater reliability; that is, different raters gave similar scores for each instrument. The inter-rater reliability for the ICER framework was not as high, perhaps because of the nature of the conceptual framework for its evidence rating matrix, which was not intended to be numerically scored.

Cohen et al. [5] evaluated the face validity of the ASCO, ICER, and DrugAbacus on the basis of four proposed logical criteria. They found several challenges pertaining to the frameworks' use of value criteria, concluding that although these frameworks "capture value in a way that is important to various audiences, they are not always logical or consistent." In part, these inconsistencies stem from problems of perspective, for example, whether and how they take the perspective of clinicians or patients.

Guiding Principles for Value Frameworks

A number of researchers and organizations have formulated lists of "good principles" that they believe should guide the development and use of value frameworks [15,16,24–28]. The principles share common themes, including: calls for transparent processes for developing frameworks and reports; consideration of patient-centered care and individualized decision making; the inclusion of a broad range of high-quality evidence; the incorporation of the broad effects of interventions such as longer term outcomes; clear statement of the intended use and audience; and the inclusion of a broad range of technologies. Some of the groups have explicitly or implicitly criticized certain frameworks for not adhering to these and other principles. For example, Sorenson et al. [28] observed that the ASCO framework lacked patient engagement in framework design and value assessment, whereas ICER provided only limited access to the economic models underlying their analyses.

Comparing the STF Recommendations with Recent Value Frameworks

In offering recommendations regarding value frameworks, we note that population/plan and individual decisions are generally made sequentially—and iteratively over time—and rely on different conceptualizations of value, as discussed in the article by Garrison et al. [13]. In a typical US health insurance setting, plan-level decisions about whether and how to reimburse new technology precede individual clinician-patient decisions about appropriate use of those technologies, with those individual decisions often informed by practice guidelines, clinical pathways, and utilization management practices. (Note that this sequence of decisions may not apply in all cases, e.g., medical devices or "medical necessity" exceptions.) Commercially insured enrollees will have made their enrollment choices before these decisions (if indeed they have a choice of insurance options from their employer), but they may choose to switch plans after a coverage decision has been made. When enrollees become patients, they choose among treatments given physician recommendations and their own preferences for the technology, as well as insurance benefit design considerations (e.g., how much they will pay out of pocket), and their own budget constraints. Importantly, plan-level decisions, such as those pertaining to coverage, tend to rely on population averages, at least within identifiable subgroups, whereas patient-level decisions encompass factors such as individuals' histories, prognoses, and preferences.

Core Recommendations

For plan-level decisions, our STF recommends that CEA be used as a starting point for underlying value constructs from the health insurer perspective. Elements that may be relevant for