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Advancing Value Assessment in the United States: A Multistakeholder Perspective

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ABSTRACT

Rising costs without perceived proportional improvements in quality and outcomes have motivated fundamental shifts in health care delivery and payment to achieve better value. Aligned with these efforts, several value assessment frameworks have been introduced recently to help providers, patients, and payers better understand the potential value of drugs and other interventions and make informed decisions about their use. Given their early stage of development, it is imperative to evaluate these efforts on an ongoing basis to identify how best to support and improve them moving forward. This article provides a multistakeholder perspective on the key limitations and opportunities posed by the current value assessment frameworks and areas of and actions for improvement. In particular, we outline 10 fundamental guiding principles and associated strategies that should be considered in subsequent iterations of the existing frameworks or by emerging initiatives in the future. Although value assessment frameworks may not be able to meet all the needs and preferences of stakeholders, we contend that there are common elements and potential next steps that can be supported to advance value assessment in the United States.

Keywords: health care decision makers, standards, value assessment, value for money.

Introduction

Rising health care costs in the United States have created significant financial burden on patients and their families, health providers, industry, and payers. New, high-priced treatments and services have faced scrutiny regarding the value they deliver in relation to their costs, especially because policymakers have accelerated efforts to shift from traditional "fee-for-service" reimbursement to "value-based" payment.

Amid these efforts, a number of professional and private organizations have put forth value assessment frameworks to define and measure the value of drugs and other therapies in a more explicit and systematic manner. Such frameworks have been introduced by the American College of Cardiology and American Heart Association (ACC-AHA) [1], American Society of Clinical Oncology (ASCO) [2], Institute for Clinical and Economic Review (ICER) [3], Memorial Sloan Kettering Cancer Center (MSK) [4], and National Comprehensive Cancer Network (NCCN) [5]. The aims of these frameworks differ—some seek to help physicians and patients make more informed, evidence-based treatment decisions, whereas others are intended to aid payer coverage determinations or price negotiations between payers and manufacturers.

Current value assessment frameworks have received praise as tools to achieve higher value health care, but have also been criticized for their limitations regarding methods, processes, implementation, and potential impacts on patients' access to care [6–11]. For example, some patients have experienced barriers obtaining new cholesterol-lowering drugs, PCSK9 inhibitors, after their (relatively unfavorable) value assessment by ICER and public debate regarding their cost [12,13]. Given their early stage of development, and their potential impact on care access and delivery, timely and ongoing evaluation of these efforts is critical to improve them and to help ensure they are implemented effectively. Such an exercise should involve all stakeholders to obtain a comprehensive, balanced, and representative assessment. In addition, we maintain that stakeholder input and, to some degree, buy-in are important to ensure the adoption and sustainability of value assessment in the United States.

To meet this aim, we convened a small group of eight stakeholders representing patient advocacy organizations, drug manufacturers, payers, and academia to explore and discuss the

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following four areas: 1) main gaps and opportunities posed by the current value assessment frameworks, 2) differences and convergences in stakeholder definitions of value, 3) key guiding principles for value assessment, and 4) potential next steps for improvement. A set of questions was developed and sent to involved stakeholders to gather their respective input across these four areas. Responses to these questions were then discussed during two rounds of meetings with all stakeholder representatives to further flesh out similarities and differences across perspectives and reach consensus on key guiding principles for value assessment.

This article offers the resulting multistakeholder perspective on value frameworks, outlining 10 fundamental guiding principles and associated strategies that should be considered in subsequent iterations of the existing value assessment frameworks or by emerging efforts in the future. This multistakeholder approach differs from the approaches of other groups, including the National Pharmaceutical Council and the Pharmaceutical Research and Manufacturers of America, which have recently put forth principles or practices for value assessment. A value assessment framework may not be able to meet all the needs of all the stakeholders, but we contend there are common elements and steps forward that can be supported and adopted to advance high-quality value assessment in the United States.

A Multistakeholder Perspective on Existing Value Frameworks

Emerging value assessment frameworks have both commonalities and differences across intended aims, methods, processes, and applications, which are largely attributable to the interests and expertise of the developing organizations and intended uses of the respective frameworks (Table 2). For example, as professional medical societies, ASCO and the NCCN designed their frameworks to enhance shared treatment decision making between patients and physicians, whereas ICER and the MSK have developed frameworks to mainly support payer coverage and pricing determinations.

These frameworks offer new tools to better understand and measure the value of new drugs and other interventions to support policy and practice and should be recognized for fostering broader conservations about these issues. Many of these efforts have strived to evolve (or plan to do so) on the basis of stakeholder input on their strengths and limitations (Table 1). Despite these steps, important issues remain that should be addressed before value assessment frameworks can be broadly implemented.

First, the existing value assessment frameworks apply a somewhat narrow and static conception of value. In particular, the elements or components of value considered across frameworks principally focus on clinical and economic outcomes. While some frameworks do take into account quality of life, severity of disease, and daily functioning, for example, patient organizations, among other stakeholders, argue that insufficient consideration is given to outcomes important to patients and their caregivers, such as personal aspirations, productivity, out-of-pocket costs, hope, convenience, and certainty of prognosis or treatment [14]. Patient perspectives on value, which are often individual and disease-dependent, can differ from those of payers and physicians in significant ways. For example, a recent study of patients with metastatic breast cancer showed that patients tend to emphasize value in terms of their personal benefit (e.g., ability to maintain rich relationships with family members) rather than in economic terms [15]. In addition, most of the frameworks fail to account for the range of benefits afforded by new or incremental innovation. For example, incremental innovation may support a new delivery mechanism, such as a patch versus a pill, that results in more sustained therapeutic drug levels, improved patient treatment adherence, and enhanced clinical choice. In particular, the industry is concerned that such advances (as well as more substantial innovations, such as curative therapies) will not be recognized sufficiently for the improvements in health outcomes and potential cost offsets they deliver over time [7]. From a payer perspective, the existing frameworks are limited in their scope by focusing narrowly on patients and individual technologies, without due account of a population or societal perspective and the continuum of patient care [16].

Second, the underlying methods used by the frameworks have not been sufficiently vetted against established standards or through broad stakeholder consensus. There is limited consistency across frameworks regarding standards for grading the quality of available evidence, evidence synthesis, costing and budget impact methods, or weighing benefits versus costs (e.g., cost-utility analysis and multicriteria decision analysis). Consequently, individual assessments may differ across frameworks, as demonstrated in the case of new treatments for multiple myeloma. ASCO calculated a fairly low net health benefit (47/130) and substantially greater monthly costs for a newer regimen (bortezomib + melphalan + prednisone) than did the previous standard of care (melphalan + prednisone) ($7042 vs. $279), but did not assess the quality of evidence [2]. The NCCN provided scores of 4 for efficacy, 3 for safety, 4 for quality and consistency of the evidence, and 3 for affordability (all out of 5) for the same three-drug regimen [17]. ICER examined six new second-line or later treatment regimens and determined that care value was low to intermediate, depending on the regimen, and that none represents good value in the long-term and therefore should be offered at a price discount of more than 75% [18]. The ICER also noted that the available evidence on the individual regimens was largely adequate, but that insufficient data exist to distinguish comparative net health benefit between them.

Furthermore, several of the present frameworks do not consider the full range of available evidence, limiting their evidence base to clinical trials, and sometimes only a single trial. Although clinical trials are often the highest quality evidence available, the resulting evidence base is often limited and can be enhanced by considering additional study designs, such as well-designed observational studies. Similarly, none of the frameworks currently consider real-world or patient-generated/patient-desired data in assessments or outline clear policies on updating assessments if new evidence arises, which may limit capturing the value of therapies in actual clinical practice and accounting for the evolving nature of innovation.

Third, the transparency of the methods and processes used by value assessment frameworks is limited. In addition to the actual frameworks themselves, the data, economic models, and underlying assumptions of individual assessments have not often been made public, which hinders interpretation of and confidence in the results. Transparency concerns extend to how therapies are selected for assessment, who is involved in the assessment, how stakeholders can engage throughout the process, and when and how stakeholder input will be considered.

Finally, insufficient attention has been directed to the implementation of value assessment frameworks in the health care system. When implemented, end users have sometimes found the output challenging or confusing because of issues such as lack of transparent methods and divergent scoring approaches. Some of these issues may disproportionately affect patients, most of whom are not well versed in clinical and health economic methods. With the exception of ICER, none of the sponsoring organizations have developed detailed guidance for how to understand or use findings, which could significantly