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Policy Perspective

As Value Assessment Frameworks Evolve, Are They Finally Ready for Prime Time?

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ABSTRACT

Background: Value assessment frameworks have emerged as tools to assist healthcare decision makers in the United States in assessing the relative value of healthcare services and treatments. As more healthcare decision makers in the United States—including state government agencies, pharmacy benefit managers, employers, and health plans—publicly consider the adoption of value frameworks, it is increasingly important to critically evaluate their ability to accurately measure value and reliably inform decision making.

Objective: To examine the evolution of the value assessment landscape in the past two years, including new entrants and updated frameworks, and assess if these changes successfully advance the field of value assessment.

Methods: We analyzed the progress of the three currently active value assessment frameworks developed by the Institute for Clinical and Economic Review, the Innovation and Value Initiative, and the National Comprehensive Cancer Network, against six key areas of concern.

Results: Value assessment frameworks are moving closer to meeting the challenge of accurately measuring value and reliably informing healthcare decisions. Each of the six concerns has been addressed in some way by at least one framework.

Conclusions: Although value assessments are potential inputs that can be considered for healthcare decision making, none of them should be the sole input for these decisions. Considering the limitations, they should, at most, be only one of many tools in the toolbox.

Keywords: cost-effectiveness, health spending, pharmaceuticals, value assessment.

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Introduction

In response to rising healthcare costs in the United States and a desired shift toward value-based care, several organizations in the United States have developed value assessment frameworks to assist patients, clinicians, and payers as they assess the relative value of healthcare treatments and services. Given the potential for such frameworks to impact payer and provider decision making as well as patient access to needed treatments, it is critical to assess whether the existing frameworks are up to the challenge of accurately measuring value and reliably informing healthcare decisions. A previous analysis applied the National Pharmaceutical Council's *Guiding Practices for Patient-Centered Value Assessment* to frameworks developed by the American College of Cardiology/American Heart Association, American Society of Clinical Oncology, Institute for Clinical and Economic Review (ICER), Memorial Sloan-Kettering Cancer Center (DrugAbacus), and National Comprehensive Cancer Network (NCCN).[1-7] This assessment determined that each framework has strengths and limitations, all have clear opportunities for improvements, and none is fully matured and ready to support healthcare decision making. It then identified 6 key areas of concern (presented individually below) that needed to be addressed to advance the field of value assessment. It should be noted that these 6 areas of concern are not meant to be wholly representative of all potential issues associated with value assessment; rather they succinctly reflect a wide range of concerns and criticisms that are also identified in and corroborated by the existing literature on value assessment.[8-10]

The past 2 years have seen an evolution of the value assessment framework landscape in the United States. First, there has been a new entrant to the field, the Innovation and Value Initiative (IVI).[11] IVI released its first open-source value platform assessment, the

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rheumatoid arthritis value tool (IVI-RA), in December 2017 and its second assessment, the nonsmall cell lung cancer (IVI-NSCLC) Value Tool in January 2019. Second, NCCN introduced categories of preference (COP) into their guidelines in September 2017.[12] Finally, ICER updated its value assessment framework in June 2017; it also developed framework modifications for ultra-rare disease treatments in November 2017.[13,14]

Other updates of note include the release of the final recommendations from ISPOR's Special Task Force on US Value Assessment Frameworks and the addition of a quantitative scoring methodology for Avalere and FasterCures' Patient-Perspective Value Framework.[9,15] Change has not been universal, however. The American Society of Clinical Oncology, American College of Cardiology/American Heart Association, and DrugAbacus have not updated their frameworks over the past 2 years, nor have they been actively conducting value assessments.

Recently, as the public policy debate on healthcare spending has intensified, payer use of value assessment frameworks has increased. In 2018, the New York Medicaid program used a cost-effectiveness estimate from the ICER's value framework to inform a coverage decision for a new cystic fibrosis therapy.[16] In addition, CVS Caremark announced a new formulary management option that will also rely on ICER's cost-effectiveness analyses.[17] Similarly, the Pacific Business Group on Health announced that it is developing a formulary for purchaser use that includes ICER's analyses as a key source of information.[18] As more and more healthcare decision makers publicly consider the adoption of value frameworks, it is important to evaluate the recent evolution of the value assessment framework landscape. Have the changes that have occurred in the past 2 years advanced the field? We examined the current frameworks actively conducting assessments—ICER, IVI, and NCCN—relative to the 6 key considerations for improvement identified in the previous article.

Key Considerations for Improvement of Value Frameworks

1. Value Assessments Should Be Transparent and Reproducible

Transparency and reproducibility are necessary for credibility and validity of assessments. This area has seen improvement over the past 2 years. For example, assessments from new entrant IVI are fully transparent. Their IVI-RA and IVI-NSCLC models can be downloaded and customized by anyone, and the release of the models and source code demonstrates to both the public and the value assessment community that introducing a fully transparent and reproducible assessment can be done.

Historically, ICER's models have not been transparent or reproducible. Several months after the release of IVI's fully transparent IVI-RA model, however, ICER introduced a pilot program to share models with manufacturers.[19] Although this is a clear step in the right direction, several limitations to ICER's approach have been noted: models should be available to all stakeholders rather than subject to restricted access; models should be fully available for use and customization rather than only available for review; and model sharing should not include confidentiality agreements that restrict the ability to share and discuss the models freely with all stakeholders.[20]

NCCN's evidence block (EB) scores and COP are assigned by a multidisciplinary panel who are subspecialists in their disease area. Even though this panel consists of highly knowledgeable experts, it is not possible for an outsider to reproduce their findings. Additionally, the driving factors behind COP categorization are not transparent. Although the panel relies on their clinical expertise to assign categories and cost is often not a factor in categorization, it is not possible to tell which categorizations are driven at least in part by cost.

Transparency limitations prevent others from replicating and validating an assessment, which can diminish both its credibility and utility.

2. Value is Dynamic and Needs to Be Considered and Captured as Such

A value assessment reflects the evidence about a treatment's cost and benefit at a specific point in time, but as evidence evolves, the value of an intervention will evolve. It is important to update assessments to incorporate new knowledge. Cancer, in particular, is an area where evidence evolves quickly and frequent updates are required. Previously, most frameworks other than NCCN did not have explicit provisions for updating their assessments; however, progress has been made over the past 2 years in this respect.

NCCN continues to regularly update their guidelines, which include EB and COP, when there is new evidence. Although it has yet to do so, IVI intends to update its assessments as new evidence becomes available, and it is currently updating its IVI-RA assessment to incorporate the latest evidence.

ICER historically had not updated reviews, a concern intensified by the fact that most of their reviews occur before a drug is on the market, when the evidence available to conduct a review is at its most limited. ICER recently established a process to begin updating some of its reviews, and to date has added a new evidence update to its existing PCSK9 Inhibitor assessment to incorporate new data and released a full condition update for plaque psoriasis to incorporate new treatments and evidence.[21] ICER will be revisiting additional conditions in 2019, including rheumatoid arthritis.

Outdated assessments that do not incorporate the most recent evidence are inappropriate to inform decision making and can lead to unwarranted restrictions that do not serve the patient.

3. Value Assessments Should Adopt Broad System Perspectives in What They Assess and How They Assess It

In the previous analysis of the frameworks, none of the assessments were taking the broader societal perspective that is recommended as a co-reference case by the Second Panel on Cost-Effectiveness in Health and Medicine (the Second Panel recommends that value assessment consider both health sector and societal perspectives).[22] A broader societal perspective includes factors like a patient's ability to work (productivity) and caregiver burden. There has been some progress toward this consideration.

IVI's approach takes a broad system perspective, demonstrating that such an approach to value assessment can be implemented. ICER has made incremental progress by including productivity in scenario analyses for its reviews and by building productivity into the base case for reviews of ultra-orphan treatments. Ideally, productivity would be built into the base case for all reviews; ICER's productivity scenario analyses are typically not highlighted in the review or the report-at-a-glance summary and are likely not considered by the end user. NCCN has not broadened its perspective, retaining a clinical decision-making lens combined with an affordability assessment, even though productivity and caregiver burden are important considerations for patients with cancer.

If factors like productivity and caregiver burden are not incorporated into an assessment, then the full value of treatments will not be adequately recognized, which could restrict access and potentially stifle incentives for innovation.

A broader perspective applies not only to the approach of an individual review, but to the approach for selecting review topics as well. Currently, value assessment frameworks are

predominantly being used to assess drugs. They should be similarly applied to all areas of healthcare.

A limited focus on drugs, without assessing the value of diagnostics, devices, surgical procedures, and other types of interventions, provides incomplete information. What is needed is a solid foundation to critically assess value in all aspects of our healthcare system.

4. Value Assessments Should Incorporate What is Important to Patients, Even If the End User for a Framework is the Payer

The patient perspective is key to a value assessment, as they are the recipients of healthcare services. Frameworks should incorporate components of value that are important to patients and engage them meaningfully in the assessment process. Over the past 2 years, some progress has been made to actively involve patients in the development and refinement of frameworks and assessments, but more can be done.

IVI worked with patients and other stakeholders to develop Partnering with Patients Principles and Commitments for its value assessment processes.[23] ICER has involved patients and patient groups to varying degrees in its reviews. The NCCN process does not currently include patients.

Working with patients is necessary, but not sufficient; patient engagement must be meaningful and impactful. It is incumbent on all groups to evaluate the success of that engagement from the patient's perspective. The National Health Council's Patient-Centered Value Model Rubric provides a tool to evaluate and guide meaningful patient engagement.[24]

Ultimately, patients are the ones who will be impacted by value-based healthcare decision making, and so it is critical to meaningfully involve them throughout the framework development and assessment process. Not involving them can lead to assessments that miss the mark on what patients value the most and what is important to them, leading to decisions that are not necessarily in their best interests.

5. A Diversity of Value Assessment Approaches That Reflect the Differing Needs of Stakeholders Should Remain; Value Assessments Should Reflect User Preferences

A diversity of assessments is needed, as no single framework with a predefined set of weighted variables can reflect the needs of disparate stakeholder groups or even the inevitable heterogeneity within a particular group. The entry of IVI into the value assessment community enhances that diversity, but meaningful diversity in the community is lacking. Currently, ICER is the only organization that is regularly conducting formal health technology assessments, generating 10 or more per year.

Model customization can support a more flexible approach, as it allows the user to conduct sensitivity analyses by changing parameters and adding new evidence. The degree of customization varies widely. IVI models are completely customizable; ICER has no user customization available nor does NCCN (although arguably an EB user can "weight" various blocks differently).

Not having a diversity of frameworks and a customizable approach can lead to decision making that may work for the hypothetical average patient but will not work for many of the patients in the real world.

6. Assessments of Value Should Be Separate from Assessments of Budget Impact and Affordability

The previous analysis emphasized the definitional differences between value, budget impact, and affordability, and the inappropriateness of including the latter 2 in an assessment of a treatment's value. Value is an assessment of net benefit relative to net cost for an individual patient: both the cost to the patient and the cost to the payer. Budget impact is a population-level measure of the number of patients who might receive a treatment multiplied by the net cost of the treatment; it does not measure whether the treatment is a good value or not. Affordability reflects ability and willingness to sustain a treatment's budget impact.

IVI and NCCN do not include estimates of budget impact in their value assessments, although NCCN does include an affordability block in the EB component of their guidelines. ICER, however, continues to hold their assessments of budget impact up against an artificial affordability threshold that it developed. Although both budget impact and affordability are important considerations, they are not measures of value. It is not appropriate for ICER to suggest that, to be of high value, the price of a treatment must be low enough to not trigger ICER's artificial affordability threshold.

Allocating resources based on an artificial affordability threshold rather than allocating them based on value can lead to inefficient use of our healthcare dollars, shifting spending away from high-value drugs and services and toward lower-value ones. This is diametrically opposed to the actual purpose of value assessment, which should be shifting spending toward high-value treatments and services and away from lower-value ones.

Conclusions

The field of value assessment has advanced in most areas. Because each consideration has been addressed in some way by at least one framework, we urge framework developers to focus on those they have not yet tackled.

Policy momentum is building around the need for accurate and methodologically rigorous value assessment. Done well, value assessments have the power to promote value in patient care and outcomes. Done poorly, they could misinform healthcare decisions and harm patient care and outcomes.

More progress is needed before the widespread adoption of frameworks for decision making. CVS Caremark and New York's Drug Utilization Review Board have chosen to use ICER's reviews to make important healthcare decisions. Of particular concern, they have each chosen to use a single cost-effectiveness point estimate from the reviews for their decisions, discarding a multitude of additional information from both within and beyond the ICER reviews.

Users must be aware that these assessments have limitations, and wholesale adoption is not wise. Although value assessments are potential inputs that can be considered for healthcare decision making, none of them should be the sole input for these decisions. Considering the limitations, they should, at most, be only one of many tools in the toolbox.

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