

Guiding Practices for Patient-Centered Value Assessment

Dan Leonard, MA, President, National Pharmaceutical Council

In response to growing concerns about healthcare costs, during the past few years, we've seen an increase in the development and use of value assessment frameworks. A variety of physician, payer, and patient-based organizations have created these frameworks as a way to measure the value of a healthcare treatment and define value through the lens of specific target audiences.

This list of organizations developing frameworks is growing, with the Institute for Clinical and Economic Review (ICER), Sloan Kettering Memorial Cancer Center (DrugAbacus), the American Society for Clinical Oncology, the American College of Cardiology/American Heart Association, the National Comprehensive Cancer Network (NCCN), FasterCures/Avalere, and the National Health Council among those that have created assessment tools.

In the United States, value assessments are a new and evolving area and have the potential to significantly affect payer coverage and reimbursement policies as well as the resulting access to treatments for patients. With this potential impact in mind, the National Pharmaceutical Council (NPC) developed a set of patient-centered guiding practices that can serve as a guidepost for framework developers and users. Establishing and adhering to guiding practices can help ensure that value assessment frameworks are effective tools for advancing patient care and achieving better outcomes for patients rather than well-intentioned but flawed tools that impede such progress.

At a high level, NPC's guiding practices outline 6 main areas of focus that should guide framework developers and assessments:

- ♦ **Assessment process:** Stakeholder input and feedback—especially from patients—should be incorporated throughout the assessment process, from the announcement of topics to be examined through the entire review cycle. Current frameworks

still vary widely in the extent to which they embed patients in the process. Patients are ultimately affected by the value-based decisions of other stakeholders, so it is critically important to engage with patients and consider their perspectives throughout the framework development and assessment processes.

As part of the assessment process, methodological experts also need to be involved. As frameworks use more advanced methods to evaluate different types of evidence, conduct extensive economic modeling, and serve more diverse users, they should revisit their mix of expertise and ways in which experts are involved in the processes. Additionally, assessments should be reviewed regularly to keep pace with continued medical innovation and updated evidence.

- ♦ **Methodology:** Assessments should focus on all aspects of the healthcare system. The majority of the focus is still on drugs and biologics, leaving stakeholders without value assessments for devices, diagnostics, surgical procedures, and other interventions. Frameworks should use established methods, as well as transparent models and assumptions, and include guidance to help users understand key drivers behind the results.
- ♦ **Benefits:** Assessments should include a broad array of factors that are important to patients and society, such as quality of life, the ability to work, and a reduction of the burden on caregivers. Assessments should also consider individual treatment effects and view the value of a treatment over the long-term horizon.
- ♦ **Costs:** All healthcare costs and offsets over time should be considered, and these costs

should be accurate and relevant to the user of the framework. There is currently wide variation among the value frameworks with respect to cost analyses and assumptions. This highlights the importance of transparency in the assumptions, evidence, and methods used, as well as recognition of their impact on the findings of value assessments and how these findings will be applied.

- ♦ **Evidence:** All the sound, high-quality evidence that is currently available should be used for the assessment. It should be gathered and synthesized in a transparent and robust manner, using accepted methods.

Unfortunately, this is an area in which some frameworks are still struggling. Frameworks rely on evidence about treatments that is available at a given point in time, which can be limited to a particular population or outcome. Evidence evolves over time, providing a greater understanding of how a product works in the real world or in patients with multiple comorbidities. While payers routinely update their coverage decisions as this new evidence evolves, ICER assessments or DrugAbacus inputs, for example, are a static representation of evidence at a point in time. ICER does not conduct routine updates to its assessments as new findings become available; in fact, it has conducted a supplemental “brief evidence update” only once. By contrast, NCCN regularly updates its assessments, which it calls evidence blocks, as new evidence becomes available. Outdated information could hurt, rather than help, patients.

- ♦ **Dissemination and utilization:** Assessments should be clearly labeled for their intended use and should be easy for users to interpret and disseminated only after they are finalized. If a framework’s results are misinterpreted, patient access to appropriate treatments could be seriously affected.

It’s notable that some of the existing frameworks and the more recently developed ones, such as FasterCures/Avalere, are incorporating these guiding practices into their value assessment frameworks and processes. Developers are recognizing that these are key components to achieving a more complete and meaningful picture of the value of a health treatment.

Importantly, there should be a broad array of frameworks in healthcare decision making. Most stakeholders understand that taking a one-size-fits-all approach and relying on a single framework to evaluate a healthcare treatment or intervention is not sufficient to inform care for diverse populations. Utilizing multiple frameworks and other decision-making tools can provide far more information as well as important and different perspectives, such as those of physicians, consumers, and especially patients.

It is becoming increasingly clear, though, that frameworks will continue to evolve and be part of the landscape. Our dialogue about frameworks in the United States will be ongoing and will require input from a variety of stakeholders. Setting forth guiding practices can help ensure that frameworks are effective tools for decision makers who support value in patient care and outcomes rather than well-intentioned but flawed tools that impede it. [ajpb](#)