



Principles for Value Assessment Frameworks

PhRMA supports the use of sound evidence for informed decision-making in health care. When designed well and used appropriately, emerging frameworks to assess the value of medical tests, treatments and health care services represent one of the many tools that can be useful to support well-informed, patient-centered health care. At the same time, it is important to ensure value frameworks are not misused in ways that impose centralized, one-size-fits-all policies, impede patients' and physicians' ability to tailor care to individual needs and preferences and hinder progress against unmet medical need.

These principles can help ensure value frameworks and assessments meet patients' needs and support continued improvement in health care. Frameworks that incorporate these principles can improve health care decision-making and the efficiency of our health care system, while frameworks that are inconsistent with these principles will make it more difficult for patients to obtain health care and treatment options that best meet their needs and discourage continued medical progress.

Background

Emerging value frameworks incorporate an assessment of evidence on clinical and economic data, and can be viewed as a subset of, or novel methods for, health technology assessment (HTA). They are designed to inform a range of different audiences and health care decisions, including treatment and prescribing decisions by patients and physicians as well as pricing or policy decisions made by private payers. A framework may be limited to or give particular priority to one of these perspectives.

Value frameworks are emerging at a time when other significant changes are occurring in health care, including: increased focus on patient- and consumer-centered care; growth in payment models that seek to incentivize health care value; growing capacity for generation of real-world evidence of value by a range of stakeholders using electronic health data; and the emergence of personalized medicine enabled by a growing understanding of genomics and capacity for storing and analyzing large volumes of electronic health data. As value frameworks emerge, it is important for them to align with these trends.

Value frameworks can be useful decision-support tools but should not be viewed as providing a single, universally applicable answer to questions about a treatment's value. Value frameworks typically emphasize one of several perspectives (e.g., payer, patient, society, or innovator) and conclusions may not apply to individual patients. In addition, as with any economic model, value frameworks involve making choices about methods, assumptions and data that can yield important differences in results depending on the choices made. This is reflected in the disparate assessments produced by different frameworks. These factors, combined with lack of consensus on best practices and inconsistency in level of transparency, underscore the need to construct and use value frameworks appropriately, as outlined in the principles below. Experience in some countries outside the U.S. illustrates how value frameworks can be used in ways that deny access to care options that clinicians and patients recognize as highly valuable.

These principles are focused on emerging value frameworks and assessments in the context of the U.S. health care system grounded in market competition. At the same time, many of these principles are relevant for HTA more generally. While HTA is traditionally focused on payer-level decision-making, value assessment frameworks may seek to inform physician and patient treatment decisions at the individual level as well as decision-making at the population level. Regardless of who is utilizing the framework, it is essential to keep the patient at the center and ensure that population-level decisions do not hinder progress against unmet medical need, or impede physician flexibility in treatment decision-making or patient access to high value care at the individual level.

The Principles

- 1. Utilize open and transparent processes for developing value frameworks and reports:** Value frameworks should be developed through an open and transparent process that includes: advance notice of priorities for assessment and scoping documents for planned assessments; opportunity for technical input from organizations with expertise in the items or services being assessed, including manufacturers when relevant; opportunity for public input on draft reports and public responses to comments received. Panels used in the development of value assessments and frameworks should be balanced and well-versed on topics under review, have relevant expertise, and should be provided materials for review in advance of meetings. Meetings should be open to the public and meeting output publicly reported.
- 2. Communicate results of final value assessments consistent with the goal of patient-centered decision-making:** Results of value assessments should be communicated after they are finalized, and in ways that support or, at a minimum, do not impede physicians and patients in tailoring decisions to the needs and preferences of the individual patient. Developers of value frameworks should make clear their process for releasing both draft and final value assessments. Communication should be consistent with standards for comparative effectiveness research communication described in statute creating PCORI.¹ Results of draft reports should not be widely disseminated or communicated as providing actionable guidance for decision-makers.
- 3. Undergo thorough validation and testing:** Value frameworks should undergo thorough and transparent validation both before and after development to ensure that they do not negatively impact health outcomes. In addition, framework output (assessment reports) should be subject to ongoing validation to ensure that accurate, reproducible findings are being generated. Frameworks also should be subject to peer-review to ensure they are consistent with well-established standards and methods.
- 4. Ensure a strong role for physicians and patients:** Practicing physicians and patients bring essential expertise and perspective, and should have a central role in the prioritization and development of value assessments to ensure they draw on physicians' clinical expertise, reflect patient values and respect patient differences.
- 5. Clearly state the intended use and audience:** The developers of value frameworks should clearly specify the intended audience of assessments and the level of decision-making they aim to support. Value assessments should incorporate design and content appropriate for the audience and intended purpose. Regardless of the type of decision-making the assessment seeks to inform, it should facilitate patient-centered care.
- 6. Prioritize patient-focused value frameworks to support individualized treatment decision-making:** The greatest opportunity for patient-centered value frameworks is through the development of more robust tools and decision aids to help physicians and patients decide which care options are most valuable to the individual. Patient-focused value frameworks should align with and support the goal of shared decision-making, and allow a patient to customize the assessment based on their individual preferences. Regardless of the level of decision-making where they are used, value assessments should not be misused at the population level in ways that impede physicians and patients from tailoring evidence-based decisions to the needs and preferences of the individual.
- 7. Use rigorous methods and make them transparent to researchers and users:** Methods for assessing value should be grounded in sound, recognized methods and be subject to meaningful and rigorous peer review. Value frameworks should provide transparency in methods to allow other organizations to replicate findings, give decision-makers confidence in the findings, and give innovators predictability in standards being used. This should include transparency in types of data used, economic models and assumptions made. Users need to understand assumptions that affect results, whether they have a strong foundation, and be able to assess the effect of alternative assumptions.
- 8. Ensure that models utilize accurate, relevant data for assessing and reporting costs and economic outcomes:** If cost or affordability information is incorporated into a value framework, it is important that the information is conveyed in a way that is accurate, appropriate, and relevant to the intended audience. Value frameworks should seek timely and accurate data on cost information, gather input from relevant stakeholders, including physicians, and provide full transparency surrounding economic models used while protecting any commercial confidential

data. Value frameworks intended to support patient-level decision-making should provide cost information relevant to the individual patient, such as out-of-pocket costs.

9. Incorporate a broad range of high-quality evidence: Value frameworks should include a broad range of rigorous and widely available scientific evidence, as incorporating only a portion of available evidence will ultimately limit the utility of the framework in practice. Any information that is potentially proprietary or commercially sensitive should be protected. Similarly, developers of value frameworks should use sound methods for synthesizing evidence.

10. Consider the broad effects of health interventions: Health care interventions can have a wide range of direct and indirect costs and benefits. Value assessment frameworks should capture these in ways that provide comprehensive, accurate information on value, and convey information relevant to the intended audience and use. Quality of life, patient-reported outcomes, survival, patient functionality and economic productivity are among the factors important to patients and society.

11. Prioritize the inclusion of longer-term outcomes: Assessments should appropriately consider both short term and long term outcomes. Assessments based only on short-term costs and benefits will likely de-value important advances, which often reveal longer-term clinical benefits and cost offsets through reduced complications (e.g., survival from cancer medicines, reduced heart attacks through management of cholesterol levels) and hospitalizations. Likewise, after the expiration of exclusivity on medicines, the treatment may be widely used by a large number of patients at a lower cost, generating benefits that should be recognized in value assessments.

12. Value progress against unmet medical needs: Value frameworks should recognize the value of progress against diseases in which there is unmet need by aligning with the processes through which that progress occurs. This includes: accounting for the inherent value of scientific and biomedical advances that add knowledge about diseases and interventions and provide stepping-stones to future advances; recognizing inherent uncertainty in the innovation cycle that often involves introduction of highly promising advances followed by ongoing research on longer-term clinical outcomes; the emergence of personalized medicine; and the step-wise nature of progress in which significant gains for patients are achieved via advances that build on one another. Current models that make conclusions based on global budgets or spending caps fall short of the goals of patient-centeredness and true value assessment, and as a result devalue many important advances against unmet medical needs.

13. Support value across the health system and continuum of patient care: Value frameworks should have a holistic, system-wide scope of work that evaluates all relevant aspects and settings of care. Consistent with the Patient-Centered Outcomes Research Institute's (PCORI) mandate for its work on comparative effectiveness², value assessment should examine the full range of health care items and services (e.g., medicines, devices, diagnostics, surgery) and the care management and delivery strategies that influence patient care. Several emerging value frameworks are intended to help guide decisions of health care resource allocation, which cannot be done in a meaningful, informed way without examining all relevant aspects of clinical care and patient management.

14. Examine patient subgroups to meet individual patient needs and optimize value: Value frameworks should consider and reflect the needs of patient sub-populations, who often respond differently to medicines based on factors such as age, genetic variation, and comorbidities. Because patient sub-populations can differ in their response to therapy, a variety of treatment options may be required to optimize treatment and provide the most clinical benefit and the greatest value. Recognizing patient heterogeneity is particularly important to ensure alignment with the emergence of personalized medicine.

15. Support availability of multiple value assessments from a range of organizations: Value frameworks seek to meet the needs of a wide range of decision-makers, and involve the evaluation of complex interventions using sophisticated and variable methods and assumptions. Decision-makers will benefit from multiple value frameworks, along with other data sources, to support their decisions and ensure the availability of relevant, timely, and high-quality reports.