In recent years, a variety of “value frameworks” have emerged—largely in reaction to rising health care costs and other perceived health system inefficiencies—with the aim of better assessing the value of health care interventions and particularly new medicines. Many of these frameworks seek to provide evidence and/or a recommendation for payers in making decisions about specific medical products and services.

At the same time, many researchers and thought-leaders are recognizing that, for value and value assessment to play a more meaningful role in health care decision-making in the U.S., better definitions and methods for assessing value must be developed. Appropriately defining and measuring the value of a health care intervention can significantly alter individual perceptions of value.

The value of an intervention depends on the outcomes it produces in a specific disease and the characteristics of the patient. As such, one important aspect of value assessment is the identification and measurement of outcomes that matter to patients, but that often are not captured in pre-registration clinical trials or lack meaningful patient engagement in their development and use. Patient-centered outcomes (PCOs) can be defined as the outcomes important to patients in the way they experience a disease or a treatment for that disease. PCOs may include a range of measures: clinical (mortality, biomarkers), patient-reported outcomes (symptoms, function, preferences), treatment-related attributes (mode of administration), resource availability and use (hospitalizations), and/or societal impacts (productivity, caregiver burden). Further, individual patients will often have preferences specific to their circumstances and inclinations. Important PCOs are often omitted from traditional approaches to value assessment methods and processes, which tend to focus on a selected subset of clinical outcomes simply because these are the endpoints studied in trials. Incorporating patient input throughout the medical product development and inclusion of PCOs.\(^1\)
As the field of value assessment evolves, it will be critical to invest in better methods and data infrastructure to identify, capture, and quantitatively incorporate PCOs in value assessment of a medical product and ensure that these analyses reflect what matters most to patients. While significant progress has been made in the field of PCO research (PCOR), there remains a need to better incorporate PCOs in the value assessment process. Initial steps to identify which PCOs are core to specific patient communities can also guide prioritization for data generation efforts.

In the shift toward a value-driven health care system, there appears to be growing consensus on the need to apply a concept of value that is reflective of the patient perspective and based on active patient participation that incorporates the patient voice throughout the medical product development life-cycle. As advances in the life-sciences industry continue to result in novel medical discoveries and promising cures for patients affected by acute, chronic, and rare diseases, it is more important than ever to ensure the field of value assessment advances with it. Until significant progress is made to better align value assessment with PCOs and the ability to capture, measure, and operationalize them, defining the true value of a health care intervention will remain a challenging endeavor. Better capture of PCOs in value assessment can also pave the way for their consideration in other decision contexts, such as innovative contracting arrangement and quality measurement.

A key challenge in accounting for patient-centered outcomes and impacts in value assessment is the lack of existing data (e.g., clinical trials, observational data sources) and data collection tools (e.g., prospective EMR data collection) to measure outcomes important to patients.

We invite submissions based on bold and creative ideas to advance methods and process associated with value assessment (and/or value elements). The PhRMA Foundation seeks papers that describe solutions to one or both of the following questions. (The total response must not exceed 3,000 words excluding title, figures and bibliography.)

1. What are potential solutions for more rapidly prioritizing and closing evidence gaps to measure treatment effects on patient-centered outcomes and impacts (e.g., using RWE and electronic health data) in clinical or economic evaluations? Include case studies and consider the role of key research organizations (e.g., PCORI, AHRQ).

2. What templates or techniques can be employed to reliably demonstrate important gaps in meaningful patient-centered outcomes and impacts?

Examples may include, but are not limited to:

- Propose a conceptual approach that allows a population-based value assessment to be tailored to individual preferences at the point of care decision-making
- Develop an approach to define a comprehensive set of PCOs to capture health care value that can be integrated into a value assessment framework
- Design a framework for identifying PCO measures, including relevant data sources, such as real-world data or qualitative research with patients
- Identify solutions to data limitations and barriers related to operationalization of PCOs, such as leveraging patient registries
- Design a value assessment methodology that accounts for patient-centered outcomes
- Propose a mechanism to collect or disseminate patient-reported data to incorporate in value assessment
- Explore how emerging innovative contracting arrangements can play a role in developing or advancing adoption of PCOs and measures
Recipients of Challenge Awards may be asked to present their winning papers at a public forum. Awards will be given in the following amounts:

- The winner will receive $50,000
- The runner up will receive $25,000
- Third place will receive $5,000

The PhRMA Foundation will not support evaluations of specific health care interventions.

**Application Process**

Candidates should submit applications no later than March 1, 2022. Details can be found at: https://www.phrmafoundation.org/awards/value-assessment-initiative/pcochallengeawards/.

**Award Expectations**

The PhRMA Foundation is committed to driving real change in health care delivery and recognizes the benefit of shared knowledge.

Award recipients are expected to publish their work. The PhRMA Foundation will facilitate broad distribution of published papers through a multi-faceted visibility campaign.

Awardees may be asked to present their winning papers in a public forum.

**Eligibility**

Award opportunities are open to all individuals and organizations with a specialization in health economics, outcomes research, clinical sciences, health care evaluation, public health, health equity or related disciplines.

Eligible applicants (or collaborators) should be affiliated with an academic institution in the United States, U.S. patient group or organization focused on improving health care in the U.S.

Collaboration across stakeholder groups and fields of discipline is encouraged.

The Value Assessment Initiative encourages new researchers to apply for funding. Researchers and collaborators who have received a Value Assessment Award or funding from the PhRMA Foundation in 2020, 2021 or 2022 are not eligible to apply.
Application Considerations

Evaluating the value of health care interventions is challenging. But, when designed well and used appropriately, tools that quantify the value of a health care treatment can inform decision-making for patients, providers and payers. There are several criteria to consider in developing solutions to drive high-quality value assessment.

**Stakeholder Engagement** A vital step to a successful shift toward a value-driven health care system is ongoing engagement with stakeholders. It is particularly important to incorporate patient perspectives and acknowledging that all individuals are future recipients of health care and are driving factors of high-quality value assessment.

**Real-World Applicability** All funded activities should generate resources, evidence or ideas that can be applied feasibly in the U.S. health care system. Variations in practice patterns or disparities in care (e.g., demographics, socioeconomic status and type of insurance) should also be acknowledged.

**Review and Validation** Research activities should be subject to systematic ongoing validation to ensure that accurate, truthful and non-misleading and reproducible findings are generated. Results should not be disseminated until validated through expert review, with input provided by all relevant and qualified stakeholders. The process of review should be well-documented and accompany the dissemination of the results.

**Patient-Centered Decision-Making** Value assessment tools create opportunities to support patient-centered decision-making if patients and other stakeholders are able to review and customize value information based on their own preferences.

**Addressing Uncertainty** Tools or frameworks that assess care value should adequately explain and address all sources of uncertainty (e.g., in parameter selection, decision process, measurement) and conduct and present relevant sensitivity and scenario analyses.

Application Components

- Descriptive title of proposed paper
- Short abstract of paper
- Full paper responding to challenge question, suitable for publication, not to exceed 3,000 words (title, figures, bibliography are outside of the 3,000 word limit)
- Applicant contact information and CV/Biosketch
- If applicable, names of other key personnel and their CV/Biosketch
- If applicable, name of affiliated or participating institution or organization

References

i See, FDA Patient Focused Drug Development (PFDD). "Patient-focused drug development (PFDD) is a systematic approach to help ensure that patients’ experiences, perspectives, needs, and priorities are captured and meaningfully incorporated into drug development and evaluation.” https://www.fda.gov/drugs/development-approval-process-drugs/cder-patient-focused-drug-development