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 is a significant challenge. Small differences in individual characteristics—such as age or health status, and personal experience—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 life-cycle, post-market real-world data collection efforts, and value assessment processes can inform the development and inclusion of PCOs.
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.

This year, the PhRMA Foundation seeks papers that describe solutions to the following question:

What approaches are needed to consistently and reliably incorporate patient-centered outcomes in value assessment for both population- and individual-level health care decision-making?

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
Award Funding Details

Recipients of Challenge Awards will be honored and asked to present their winning papers at a public forum in 2021. Awards will be given in the following amounts:

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

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

Application Process

The Challenge Award application process has two stages.


2. Candidates who submit a Letter of Intent will be informed by August 10, 2020, whether they should submit a full paper describing their response to the challenge question. Papers are due September 15, 2020, and will be evaluated by a panel of qualified reviewers.

Award Expectations

The PhRMA Foundation is committed to driving real change in health care delivery and recognizes the benefit of shared knowledge. Therefore, the Foundation will establish a Value Assessment Research Network to encourage collaboration and dissemination of findings borne out of the program.

Recipients of all PhRMA Foundation awards under the Value Assessment Initiative will become members of the Network and be asked to participate in periodic calls or in-person meetings to discuss and drive advancement in the field.

The PhRMA Foundation will host a public forum in 2021 to highlight activities funded by this program. Awardees must be willing and available to present their winning papers at this forum.

Eligibility

Award opportunities are open to all individuals and organizations with training in health economics, outcomes research, clinical sciences or health care evaluation. Eligible applicants should hold an advanced degree in a field of study logically or functionally related to the proposed activities.

Collaboration across stakeholder groups and fields of discipline is encouraged.
Letter of Intent and 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. Recommendations for patient engagement processes are made available by the National Health Council.

**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. For example, the second panel on cost effectiveness in health and medicine recommends that all potential consequences of care should be presented in a transparent and disaggregated form, such as in an “impact inventory table”. Additionally, all criteria should be quantified and included in assessments if possible.

**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.

**Submission Components**

This program is requesting that prospective candidates submit a letter of intent prior to the submission of a paper. The letter of intent should include the following:

**Letter of Intent Components:**

- Descriptive title of proposed paper
- Applicant contact information
- Names of other key personnel (if applicable)
- Applicant(s) CV or biosketch
- Affiliated or participating institutions (if applicable)
- Proposed response to challenge question, not to exceed 600 words

Those whose submissions are selected will be notified by August 10, 2020 and asked to complete a full paper. Instructions for submission will be provided.

**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