

# PCORI's Expanded Mandate Creates New Opportunities for Value Assessment Research

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In recent years, a growing chorus of patients, providers, and health economics researchers has been calling for a new approach to value assessment that incorporates patient-informed elements of value alongside the traditional measures of outcomes and associated costs. Identifying and measuring these value elements, and factoring them into economic evaluations of medical treatments, have been the work of a growing community of researchers seeking to merge patient centricity with economic determinations of treatment value. The importance of achieving greater patient centricity in value assessment has grown more urgent in recent months as thought leaders and policy makers put forth proposals for new value assessment research and use in the United States.<sup>1</sup>

When originally established in 2010, the Patient-Centered Outcomes Research Institute (PCORI) was positioned to play a role in funding health technology assessment: Congress granted it significant funding, explicitly included systematic reviews in its mandate, and authorized it to coordinate its work with the needs of payers as a key customer. At the same time, some elements of its mandate limited its impact, particularly the prohibition of including cost-effectiveness analysis in funded research.<sup>2</sup> Although PCORI-supported research could look at how medical interventions compare in improving health outcomes, there has been little targeted effort to drive clinical comparative effectiveness research or inform efforts to better understand intervention value.

Congress' reauthorization of PCORI at the end of 2019 created more opportunities for PCORI to play a leading role in support of patient-centered value assessment. The latest reauthorization legislation expanded PCORI's mandate, enabling it for the first time to fund studies that look at the "potential burdens and economic impacts of the utilization of medical treatments, items, and services on different

stakeholders and decision makers."<sup>3</sup> This effectively broadened the range of patient-centered outcomes data that can be collected to include "medical out-of-pocket costs, including health plan benefit and formulary design, nonmedical costs to the patient and family, including caregiving, effects on future costs of care, [and] workplace productivity."

To give clarity to this new mandate, PCORI recently outlined 4 new principles that will serve as a point of reference to the organization as it develops future guidance surrounding its support of research that captures economic impact and cost data.<sup>4</sup> These are:

1. PCORI-funded research may consider the full range of outcomes important to patients and caregivers, including burdens and economic impacts.
2. PCORI-funded research may consider the full range of outcomes relevant to other stakeholders, when these outcomes have a near-term or longer-term impact on patients.
3. The collection of data on burdens and economic impacts of treatment options must be appropriate and relevant to the clinical aims of the study.
4. Beyond the collection of burden and economic impact data, PCORI may support the conduct of certain types of economic analyses as part of a funded research study to enhance the relevance and value of this information to health care decision makers.

In short, PCORI's new mandate permits support for data collection on a wider range of outcomes that include the economic impacts of health care interventions. So, although cost-effectiveness analysis studies (or, specifically, research that employs measures of dollars per quality-adjusted life-year) remain well out of scope for PCORI, these new principles present great opportunities for those working in the value assessment

space to build on PCORI-sponsored research on burden and economic impact to inform conversations about treatment value.

The first 2 principles outline which cost and economic impact data can be collected as part of PCORI's new mandate and how they pertain to patients, caregivers, and other stakeholders. By acknowledging that burdens and economic impacts of care options significantly affect health outcomes, PCORI is further broadening the scope of patient-centered outcomes research. Outcomes that reflect patient burdens (out-of-pocket costs, costs related to transport to settings of care) and caregiver burdens (hours spent and/or wages forgone from providing care) may now be considered as a part of the "full range" of outcomes on which researchers can collect data. This shift presents a major opportunity to further develop patient-derived value elements, which can then be incorporated into economic evaluations of health care treatments. Recent external efforts to develop core sets of patient-derived value elements have yielded new insights into what it is that patients value in a treatment and what they prioritize in their decision-making.<sup>5</sup>

PCORI's new mandate also allows support for research that examines how health care interventions affect different stakeholders in the health care delivery system—laying the groundwork for outsiders to develop value frameworks that make use of these data. Multicriteria decision analysis (MCDA) frameworks that capture and weigh value elements that are important to patients and a variety of stakeholders could benefit from this.<sup>6</sup> MCDA offers an opportunity to systematically weigh traditional value elements (cost, clinical outcomes) alongside nontraditional elements (patient-reported outcomes, impacts on family and caregivers) to arrive at a more comprehensive understanding of value.

Examples of outcomes newly permitted for collection include measures such as treatment costs (staff time, medication costs, changes in dosage), costs associated with utilization changes (changes in length of stay, shift in care site), and even employer/workforce burdens (absenteeism, reduced productivity). Using MCDA, these outcomes could then be weighted and quantified by those looking to conduct research that informs payer and provider decision-making.

The third principle outlined by PCORI holds that the collection of economic impact data is required to be directly relevant to the specific clinical aim of the study. That is, economic data collection must follow a specific comparative effectiveness research aim, and economic data cannot be studied (alone) as the sole purpose of the analysis. This presents a golden opportunity for those working in value assessment to build on the comparative effectiveness framework that PCORI-supported research can give us to engage in sound, patient-centered value assessment.

For example, our team at the University of Maryland's

Patient-Driven Values in Healthcare Evaluation (PAVE) Center recently developed a model to quantify the value of direct-acting antivirals (DAAs) to treat hepatitis C virus using a patient-centered approach.<sup>7</sup> Aiming to address a perceived shortcoming of traditional comparative evaluations of DAAs, which often neglect to account for the challenges patients face beyond virologic care, our model incorporated 2 new treatment outcomes—infected life-years and workdays missed—and found that these interventions were cost-effective from the health sector perspective and cost-saving when including nonhealth costs.

This analysis was developed using data from a patient stakeholder advisory board that was initiated with a grant from PCORI. Although PCORI did not play a role in developing the actual model, it did lay the groundwork for my team to build it independently.

Finally, PCORI's fourth principle acknowledges that some economic analysis could be permitted as long as it is limited to enhancing the usability of cost burden and economic impact data for comparative effectiveness research. A study that requires a cost estimate of an emergency department visit or an elective surgery, for example, would be permitted to include a very limited analysis of collected data to arrive at a cost estimate for those 2 interventions. Although this practice certainly falls well short of cost-effectiveness analysis, it nonetheless may permit the development of rough standard measures that can later be used in value assessment models for specific interventions.

Stakeholders are largely aligned in the belief that health outcomes used in research should be derived from patients and that patients should be actively engaged in these exercises. Armed with a new mandate, PCORI has an opportunity to further explore the impact and burdens of treatment cost on patients. It is up to the value assessment community to pick up where PCORI leaves off and connect these outcomes back to value.

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