Principles for the Consideration of the Full Range of Outcomes Data in PCORI-Funded Research

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PCORI’s Principles for the Consideration of the Full Range of Outcomes Data

In September 2020, the Patient-Centered Outcomes Research Institute (PCORI) released proposed Principles for the Consideration of the Full Range of Outcomes Data (hereafter referred to as the Principles) for public input in response to the authority provided to PCORI in its reauthorizing legislation. PCORI solicited public comments through a variety of sources, including a 60-day period for written comments, two PCORI-hosted webinars, and individual stakeholder discussions. PCORI appreciates the feedback from patients, caregivers, and stakeholders across the healthcare community who provided input for the consideration of burden and economic impact data in PCORI-funded research. Commenters’ valuable insight informed the Principles and also highlighted opportunities where PCORI can further advance the implementation of this new legislative authority and support the needs of patients, caregivers, and other stakeholders.

This document outlines the Principles that PCORI will apply in fulfilling the requirements of its authorizing law to consider the full range of outcomes data—including, as appropriate, potential burdens and economic impacts related to the utilization of healthcare services—in PCORI-funded research. These Principles will inform PCORI’s development of additional guidance to potential applicants for PCORI funding on what is included in “the full range of clinical and patient-centered outcomes relevant to, and that meet the needs of, patients, clinicians, purchasers and policy-makers” consistent with our authorizing law as amended in 2019.

In addition to comments on the proposed Principles, PCORI received valuable input regarding the importance of continuing support for robust engagement between diverse patients, caregivers, stakeholders, and the research community; opportunities for PCORI to play a leading role in advancing scientific methods related to the collection and consideration of burden and economic impact data; and opportunities to inform discussions on addressing healthcare costs and value. Further, PCORI received input on specific focus areas for PCORI’s ongoing activities related to the consideration of the full range of outcomes, many of which are referenced in this document.

2 Webinar 1 and Webinar 2
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What Is in the Law?

PCORI’s authorizing law was amended by PCORI’s reauthorization legislation\(^3\) to include a new obligation. As part of PCORI’s duties to carry out its research agenda, PCORI-funded research will, as appropriate, now consider the full range of outcomes data:

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“(F) CONSIDERATION OF FULL RANGE OF OUTCOMES DATA. —Research shall be designed, as appropriate, to take into account and capture the full range of clinical and patient-centered outcomes relevant to, and that meet the needs of, patients, clinicians, purchasers, and policy-makers in making informed health decisions. In addition to the relative health outcomes and clinical effectiveness, clinical and patient-centered outcomes shall include the potential burdens and economic impacts of the utilization of medical treatments, items, and services on different stakeholders and decision-makers respectively. These potential burdens and economic impacts include medical out-of-pocket costs, including health plan benefit and formulary design, non-medical costs to the patient and family, including caregiving, effects on future costs of care, workplace productivity and absenteeism, and healthcare utilization.”\(^1\)
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This provision clarifies that in addition to relative health outcomes and clinical effectiveness, relevant outcomes may also include the potential burdens and economic impacts of the utilization of medical treatments, items, and services. The Principles in this document will inform applicants about PCORI’s current interpretation of this provision and PCORI’s current thinking about where to develop more specific guidance for applicants on how to incorporate the consideration of the full range of outcomes data—clinical effectiveness, quality of life, and other patient reported outcomes, as well as burden and economic impact—in their research proposals and studies.

While PCORI’s authorizing law does direct PCORI-funded research to capture, as appropriate, economic impact and cost data, it still limits what PCORI may fund, what PCORI can develop, and what may be included in reports of research findings. The authorizing law specifies that PCORI “not develop or employ a dollars-per-quality adjusted life year (or similar measure that discounts the value of a life because of an individual’s disability) as a threshold to establish what type of health care is cost effective or recommended.”\(^4\) Moreover, PCORI is not

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permitted to “mandate coverage, reimbursement, or other policies for any public or private payer.” The authorizing law further directs PCORI to “ensure that the research findings . . . do not include practice guidelines, coverage recommendations, payment, or policy recommendations.” PCORI has implemented these mandates in its funding announcements and guidelines by stating that applications for funding that seek to conduct a formal cost-effectiveness analysis or that are part of efforts to develop coverage recommendations, payment or policy recommendations, or clinical practice guidelines will not be eligible for PCORI funding. This will remain the practice of PCORI moving forward.

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6 Social Security Act, Section 1181 [42 USC 1320e (d) (8)(A)(iv)]. (1935)
Results

PCORI’s mission is to promote the ability of patients and the public to make informed healthcare decisions to improve their desired health outcomes and to improve healthcare delivery and outcomes, by producing and promoting high-integrity, evidence-based information generated through research guided by patients, caregivers, and the broader healthcare community.

To achieve those goals, PCORI carries out its statutory mandate to fund comparative clinical effectiveness research (CER) that takes into account patient-centered outcomes and preferences. Patients and consumers make choices about health care every day—but too often lack the evidence to choose the care that best meets their needs. For patients, the gaps in available information can result in greater burdens on them, their families, and the healthcare system. When patients and their caregivers have access to credible, evidence-based information, they can make healthcare choices that are more relevant and useful. Such informed decisions can improve health outcomes for patients, better target patient-centered and personalized care, and lead to a more effective and efficient healthcare system.

In addition to having a better understanding of the clinical benefits and harms, as well as the impacts on patient-centered outcomes, of various treatment options, patients and healthcare consumers are increasingly faced with the need to understand the substantial range of burdens and economic implications of those choices, as these can have real and important effects on patients’ broader well-being. The economic impacts on patients and families include direct expenses, particularly out-of-pocket medical expenses; however, other costs, such as caregiver time and a patient’s time away from work, are equally relevant. These types of impacts, important from the patient perspective, are often overlooked. Efforts to assess them are undertaken sporadically rather than systematically—and not routinely assessed in the context of research that is designed to assess the clinical outcomes of an intervention. Having a better understanding of the potential burdens and economic impacts of care options may influence how patients and caregivers weigh their choices.

Although broadening the consideration of evidence to include information on burdens and economic impacts that directly affect patients is consistent with PCORI’s patient-centered mission, the amendments to PCORI’s authorizing law apply more broadly than to the impacts on patients alone. PCORI is charged with, as appropriate, considering the potential burdens and economic impacts of healthcare utilization on “different stakeholders and decision-makers.” Specifically, PCORI is charged with considering the full range of outcomes that meet the needs of “clinicians, purchasers, and policy-makers in making informed health decisions,” in addition
to those that meet the needs of patients. Impacts on other stakeholders and decision makers can affect patient outcomes and patient well-being directly. Additionally, a robust evidence base can help these stakeholders make informed decisions about how to better organize healthcare or clinical workflows. This evidence might inform how an employer determines what options best address workplace productivity and absenteeism; or a clinic’s decision on the best way to meet a patient’s health needs; or help policymakers make more informed decisions about healthcare services. The updated charge to PCORI underscores the important role PCORI can play in providing information on potential burdens and economic impacts that will support patients, their caregivers, and other stakeholders in making informed healthcare decisions that account for such impacts.
Principles

This section highlights the key Principles that PCORI will follow in developing guidance to applicants and to merit reviewers for use when assessing applications for funding.

Principle #1: Considering the full range of outcomes important to patients and caregivers, including potential burdens and economic impacts, is central to PCORI-funded research.

Patient and caregiver priorities have always guided PCORI’s research agenda. Because burdens and economic impacts of care options significantly affect patients and their caregivers, PCORI seeks to incorporate those factors within PCORI-funded research. The goal of doing so is to provide information on the costs and impacts of treatments and services to patients and caregivers. A better understanding of the potential burdens and economic impacts of treatment options for patients and caregivers will inform not only their decisions but also those made by payers, health systems, and providers.

PCORI requires that patients be engaged in the research it funds—not as participants but as partners who help determine what to study and how. This tenet of PCORI-funded research is especially important when considering potential burdens and economic impacts important to patients and their caregivers. Patient engagement is critical not only in identifying important outcomes but also in understanding how to capture the relevant costs and burdens, as they may vary depending on many factors, such as insurance coverage. PCORI will continue to seek and support the active engagement of patients and caregivers in PCORI-funded research, including when identifying the burden and economic impact outcomes relevant to them that may not be traditionally or systematically measured.

Examples of Elements of Burden and Economic Impact Important to Patients and Caregivers

- **Patient burden**
  - Time in hospital
  - Time away from work or usual activities
  - Cost/time for transport
  - Childcare and eldercare costs
  - Out-of-pocket costs (copays and deductible; items not covered such as drugs or care providers)

- **Caregiver burden**
  - Hours spent caregiving
  - Forgone wages
Principle #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.

Stakeholders such as payers, employers, health systems, clinicians, and other decision makers responsible for designing health plans, formulary decisions, or health system improvements make decisions that have near- and long-term impacts on patients. They also benefit from a robust evidence base on the clinical and economic impacts of healthcare treatments and services to inform their decisions. Therefore, under the new authority, PCORI is charged with, as appropriate, considering the potential burdens and economic impacts of healthcare utilization on these “different stakeholders and decision-makers.”

Burdens and impacts that may inform stakeholders’ decisions include, for example, costs associated with treatments or other interventions, or with healthcare utilization resulting from these treatments or interventions. Components of such costs may include a range of relevant resource use, such as staff time (wages and benefits), costs of diagnostic tests, and medical supplies. In many cases, the costs of an intervention will involve “program costs,” such as training and supervising staff or purchasing needed equipment.

Examples of Elements of Burden and Economic Impact Important to Healthcare Stakeholders

- **Cost of treatment/intervention**
  - Staff time
  - Costs of medication
  - Changes in medication dosage
- **Utilization**: Without associated costs
- **Costs associated with utilization**
  - Costs of visits of different types (e.g., emergency department, primary care), specific to care site
  - Costs of hospital stay
- **Costs of establishing/implementing new intervention (program costs)**
- **Employer burden**
  - Absenteeism
  - Reduced productivity from presenteeism

To ensure that the data being collected are appropriate and relevant, applicants should address why the economic outcomes—such as impacts on productivity or costs of implementing a specific program—are important for informing stakeholder choices that will affect patient outcomes.

One clear example of when collection of economic data may be important is in studies where two options are equivalent with respect to clinical and patient-centered outcomes. In these circumstances, burdens or economic impacts may provide important additional information for decision making. For example, if a study establishes that health outcomes for patients receiving in-person specialty care are not better than those receiving these services through telemedicine, decisions about establishing these telemedicine programs may well turn on other factors. If the telemedicine program costs less to deliver, or if it provides
substantial benefits to patients in terms of time saved in travel to in-person visits, then this information may prove valuable to decision makers at all levels—from payers to health systems administrators to patients.

The authorizing law, as amended, directs PCORI-funded research to capture, as appropriate, burden and economic impact data. Following the example above, PCORI-funded research can provide that data as a contribution to the evidence stakeholders have available to conduct analyses to inform their decisions. Different stakeholders can use different types of analyses, such as return on investment analyses, to inform their decisions, and they can draw upon information regarding patient burden and economic impact collected by PCORI-funded research in a variety of ways.

**Example 1**

Treatment A and Treatment B might decrease numbers of emergency department (ED) visits or length of hospital stay. If so, the investigator is likely reporting on these healthcare utilization outcomes. PCORI encourages investigators to capture the cost associated with the ED visits or hospital stays observed, as part of the study.

**Example 2**

Consider a trial that is examining the comparative effectiveness of two asthma management programs and to do so, is putting these two programs in place in study clinics. If the program requires training staff and updating the electronic medical record system with prompts, then the investigator may wish to capture these implementation costs.

**Principle #3: The collection of data on potential burdens and economic impacts of intervention options must be appropriate and relevant to the clinical aims of the study.**

PCORI appreciates the need for information on the full range of outcomes, including on the burdens and economic impacts of various intervention options and services, when making healthcare decisions, and PCORI encourages the collection of these data in PCORI-funded research. PCORI’s foremost mandate, as dictated in our authorizing law, is to support the conduct of comparative CER. In recognition of this priority, PCORI does not require the collection of data on burdens and economic impacts of intervention options across all PCORI-funded research.

PCORI encourages investigators to capture appropriate and relevant cost burdens and economic impacts associated with the *impact of an intervention* (Example 1) for two or more alternative approaches that are studied within the context of CER. Additionally, when capturing the *costs of the intervention* contributes valuable and relevant information, investigators should capture this information. Investigators may also capture the costs associated with
implementing an intervention when these reflect real-world costs of relevance to potential adopters (Example 2).

PCORI does not fund studies for which cost and economic impacts are the primary outcome. Further, PCORI does not support studies that conduct cost effectiveness analysis. However, as indicated in Principle #1, PCORI has a special interest in patient and/or family burden. For this reason, PCORI welcomes and will support studies that directly compare patient and/or family cost burden (e.g., out-of-pocket costs, time costs associated with an approach, financial toxicity) as part of secondary outcomes.

In all research that it funds, PCORI expects that study outcomes should be relevant and important to patients and other stakeholders. To meet this expectation, PCORI requires applicants to engage relevant stakeholders in the formulation of the research question and the development of the study design, as well as the identification of outcomes to measure. This approach is intended to ensure that PCORI-funded research will provide evidence that is ultimately relevant and applicable for the end user; it also seeks to avoid the unnecessary capture of data that are not relevant to the aims of the study and may not be beneficial to the goals of the research. This same expectation should apply when considering whether and which potential burden and other economic impact data a research study should capture.

When considering data collection on burdens and economic impact, applicants should consider the feasibility of capturing these data types when submitting funding applications. This includes considering the availability and accessibility of relevant data, the added burdens that capturing these data may impose on study participants, ensuring that data are captured from reliable sources, data generalizability to real-world practice, and the potential limitations of the data.

When reviewing an application’s proposed collection of economic impact and cost data, PCORI will consider whether the applicant is leveraging the unique opportunities afforded by their comparative CER to collect this information; that is, the collection of economic impact and cost data should directly follow from and relate to the specific research study.
**Area of Particular Interest to PCORI**

Addressing health disparities and health equity continues to be a priority in PCORI-funded research. Cost burdens, both direct and indirect, associated with other social risk factors and social determinants of health have a direct impact on health disparities and inequities within our healthcare system. PCORI will continue to support patient-centered outcomes research that addresses and eliminates these challenges. Therefore, when considering relevant burdens and economic impacts, PCORI-funded research should consider the inclusion of outcomes that capture social risk factors and social determinants of health.

Before developing guidance and potential methodology standards, PCORI will seek opportunities to engage with patients, caregivers, and stakeholders across the healthcare community to further define this priority.

**Principle #4: PCORI may support the conduct of certain types of economic analyses, as part of a funded research study or independently, to enhance the relevance and value to patients and other stakeholders of information PCORI-funded investigators collect on potential burdens and economic impacts.**

The language of the authorizing law, as amended, charges PCORI with consideration of the full range of “outcomes data”—that is, the consideration of data relevant to potential burdens and economic impacts of healthcare interventions, as appropriate. As such, and described in the Principles above, PCORI-funded CER can include capturing data on these impacts.

PCORI understands that to facilitate the consideration of the full range of data, investigators may propose to extend their activities beyond the simple collection of existing data to the measurement of cost elements and/or to the conduct of certain types of economic analyses. For example, a study might collect data on a variety of different cost components that factor into the delivery of an emergency department (ED) visit for a patient with a suspected cardiac event: costs of staff time for clinicians and support staff, costs for use of the ED bed (e.g., linens, cleaning), oxygen, cardiac monitoring, and so on. However, the collection of these data may be more relevant—and rendered more useful—if they are summarized in a total estimated cost per visit. Developing this estimate might require analysis, for example, to reflect median use of supplies and staff time. This type of analysis is consistent with the intention of PCORI’s authorizing law. As relevant, PCORI applicants should ensure that their proposed capture of economic impact includes such analyses and that their report of study findings includes a presentation of the results.
There are, however, statutory limits on the types of analyses that PCORI can fund. In accordance with its authorizing law, as amended, PCORI is prohibited from funding research that includes practice guidelines or coverage, payment, or policy recommendations and developing or employing a dollars-per-quality adjusted life year threshold. Thus, investigators seeking PCORI funding should not propose aggregating findings on health outcomes with findings on economic impacts—as in a cost-effectiveness ratio.

PCORI understands the utility of certain types of analyses that may fall beyond the scope of its mission and the parameters of its authorizing law. PCORI’s limitations on this type of analysis extend only to studies conducted with PCORI funding. Investigators may choose to seek additional funding from other funders to support further analysis. Similarly, the prohibitions against the development of practice guidelines, coverage, payment, or policy recommendations in PCORI-funded research findings apply only to work funded by PCORI. PCORI-funded research may be used by others to inform such products.
Ongoing Activities

Beyond informing the development of these Principles, respondents during the public comment period highlighted opportunities where PCORI could further advance the implementation of its authority to consider, as appropriate, the full range of outcomes data and support the needs of patients, caregivers, and other stakeholders.

Patient, Caregiver, and Other Stakeholder Engagement

Aligned with PCORI’s mission, commenters noted the importance of diverse and ongoing patient and stakeholder engagement at both the organizational and individual-project levels. PCORI fully endorses this goal and will continue to build on efforts to meet the needs of our patient and stakeholder communities.

PCORI Guidance for the Research Community

Commenters noted the need for additional guidance for future applicants and funded investigators on key topics and questions related to this new domain of potential research activity. Examples include clarification on the types of economic analyses permitted in PCORI-funded research; criteria for research where the collection of cost data would be required, encouraged, or not expected; and the types of expertise expected or required for project personnel undertaking cost data collection. Although PCORI has provided preliminary guidance within its Cycle 1 2021 funding opportunities, PCORI understands the need for further clarity on these and other topics and will develop further guidance for future applicants. In the interim, information will be provided through PCORI funding announcements as they are released and in FAQs posted on PCORI’s website.

Methodology Standards

PCORI appreciates commenters’ support and desire for PCORI to play a leading role in advancing rigor and standards when conducting research that considers the full range of outcomes data, including those on the potential burdens and economic impacts of the utilization of medical treatments, items, and services on different stakeholders and decision makers. Specific areas where commenters noted gaps in standards include but are not limited to the following:

- Standardizing the collection of burden and economic impact data, especially as it relates to patient-centered measures that are not traditionally or systematically measured
- Factoring in social risk factors and social determinants of health when considering potential burdens and economic impacts
- Reporting of results, including the identification of data limitations
As directed in PCORI’s authorizing law, as amended, PCORI’s Methodology Committee is responsible for developing and improving “the science and methods of comparative clinical effectiveness research by developing and periodically updating” methodological standards for research. This committee is thus well suited to undertake efforts to develop “methodological standards” to more fully inform PCORI on how its funded research should consider and capture relevant data pertaining to the potential burdens and economic impacts in the conduct of their research. The process for the development and update of methodological standards will be consistent with the transparency requirements of PCORI’s authorizing law, which provides for a public comment period prior to the PCORI Board of Governor’s adoption of methodological standards developed and updated by the Methodology Committee.

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8 PCORI Methodology Standards
Summary

PCORI appreciates the wide-ranging and informative input received in the development of the Principles through the public comment period. We were particularly pleased to have had this opportunity to seek additional meaningful perspectives and gratified both by the commenters’ constructive suggestions for the proposed Principles and the identification of a number of key areas for PCORI to consider on a strategic and ongoing basis as we continue to carry out our work.

PCORI’s goal is to implement the authority to consider, as appropriate, the full range of outcomes data in a timely way, providing additional guidance for applicants and the broader research and stakeholder communities we serve. In pursing this goal, PCORI recognizes that the processes and practices developed will evolve based on experience and additional input. As part of this effort, and respecting PCORI’s legislative limitations, we will seek opportunities to advance and support discussions related to a patient-centered approach to addressing healthcare value and rising healthcare costs and remain alert to how our work informs that of others. As commenters suggested, PCORI will also consider the best ways to monitor how PCORI-funded data on burdens and economic impacts are used, including attention to any unintended uses of such information that may contribute to limitations on patients’ access to care.