PROPOSED

Consideration of the Full Range of Outcomes Data: PCORI's Principles for Public Comment

PCORI's Proposed "Principles" for the Consideration of the Full Range of Outcomes Data

This draft document outlines the principles PCORI is proposing to apply when meeting its statutory mandate to consider the full range of outcomes data – including, as appropriate, potential burdens and economic impacts related to the utilization of health care services – in PCORI-funded research. These principles will serve as a point of reference for PCORI, as a basis for developing future guidance to potential applicants for PCORI funding on what is included in "the full range of clinical and patient-center outcomes relevant to, and that meet the needs of, patients, clinicians, purchasers and policy-makers" consistent with our authorizing law.

PCORI will solicit feedback from stakeholders across the healthcare community on the principles in this document and to inform how PCORI should implement them, while ensuring that PCORI-funded research continues to be patient-centered and impactful.

What is in the Law

PCORI's authorizing law was amended by PCORI's reauthorization legislation[1] to include a new mandate for PCORI. As part of PCORI's duties to carry out its research project agenda, PCORI-funded research will now consider the full range of outcomes data:

> "**(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."[2]

[1] Further Consolidated Appropriations Act of 2020; Pub. L. No. 116-94 (2019), H.R. 1865.
[2] Social Security Act. Section 1181 [42 U.S.C. 1320e] (d)(2)(F)

The principles in this document will inform applicants on PCORI's interpretation of this mandate and on PCORI's direction in developing guidance for applicants on incorporating the consideration of the full range of outcomes data in their research proposals and studies.

While the authorizing law does direct PCORI-funded research to capture, as appropriate, economic impact and cost data, PCORI's authorizing law still limits what PCORI may fund, what PCORI can develop, and what may be included in reports of research findings. The authorizing law includes the limitation 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."[3] Moreover, PCORI is not permitted to "mandate coverage, reimbursement, or other policies for any public or private payer."[4] The authorizing law further directs PCORI to "ensure that the research findings . . . do not include practice guidelines, coverage recommendations, payment, or policy recommendations."[5] PCORI has implemented this mandate in its funding announcements and guidelines by stating that applications for funding that seek to conduct a formal cost-effectiveness analysis or whose findings will result in coverage recommendations, payment or policy recommendations, or clinical practice guidelines will be non-responsive. This will remain the practice of PCORI moving forward.

Why is Information on a Fuller Range of Outcomes Useful?

PCORI's vision is that patients and the public should have information they can use to make decisions that can improve their desired health outcomes. And PCORI's mission is to help people make informed healthcare decisions and improve healthcare delivery and outcomes, by producing and promoting high-integrity, evidence-based information that comes from 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 that takes into account patient-centered outcomes and preferences. Patients and consumers make choices about healthcare 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. Ensuring patients and their caregivers have access to credible, evidence-based information helps them make healthcare choices that are more relevant and useful to them. This 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 and the impacts on patient-centered outcomes of various treatment options, patients and consumers of health care are increasingly faced with the need to understand the economic burdens and impacts of those choices, as these can have real and important impact on patients' broader well-being. The economic impacts on patients include direct expenses, particularly out-of-pocket medical expenses. But they also include burden on a caregiver or time away from work. These impacts, important from the patient perspective, are often overlooked. Efforts to assess these impacts are undertaken sporadically rather than systematically, and it is seldom the case that they are assessed in the context of research that is designed to assess the clinical outcomes of an intervention.

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[3] Social Security Act. Section 1182 [42 U.S.C. 1320e-1] (e)
[4] Social Security Act. Section 1182 [42 U.S.C. 1320e] (j)(1)(A).
[5] Social Security Act, Section 1181 [42 USC 1320e (d) (8)(A)(iv)]

Although broadening the consideration of evidence to include information on burdens and economic impacts directly affecting patients is highly 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. But further, these stakeholders require evidence to make informed decisions about how to better organize healthcare or clinical workflows. It 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 patient's health needs; or help policymakers make more informed decisions about health care services. The updated charge underscores the important role PCORI can play on providing information on economic costs and burdens that will support patients, their caregivers, and other stakeholders in making informed healthcare decisions that take account of their impacts on patients.

Principles

This section highlights the key principles PCORI proposes to follow when providing guidance to applicants, and when reviewing applications for funding.

Principle #1: PCORI-funded research may consider the full range of outcomes *important to patients and caregivers*, including burdens and economic impacts.

Patient priorities have always guided PCORI's research agenda. And as burdens and economic impacts of care options significantly impact patients and their caregivers, PCORI will incorporate those factors in our funded research. The goal of doing so is to provide information on the costs of treatments and services for patients. A better understanding of the burdens and economic impacts of treatment options on patients not only informs patient and caregiver decisions, but also those made by payers, health systems and providers.

Potential Burdens and Economic Impacts Important to Patients and Caregiver

- **Patient burden:**
  - Time in hospital
  - Time home from work or usual activities
  - Cost/time for transport
  - Childcare costs when seeking care
  - Out-of-pocket costs (copays and deductible; items not covered such as drugs or care providers)
- **Caregiver burden:**
  - Hours spent caregiving
  - Foregone wages
- **Utilization:** Without associated costs.
- **Capture all substituted utilization/cost.**

PCORI requires that patients be engaged in the research we fund, not as subjects but as partners who help determine what to study and how. This tenet of PCORI's research is especially important when considering burdens and economic impacts important to patients and their caregivers. Patient engagement is not only critical to the identification of important outcomes, but also in understanding how to capture the costs and burdens that are relevant to them, as those may vary depending on many factors, such as insurance coverage.

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, and other decision-makers responsible for designing health plans, formulary decisions, or health system improvements make decisions that have near-term and long-term impacts on patients. They also benefit from a more robust evidence base on the clinical and economic impacts of healthcare treatments and services to inform their decisions. Therefore, under the new mandate, 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 on stakeholders that may inform their decisions include, for example, costs associated with treatments or other interventions, or with changes in utilization resulting from these treatments or interventions. Components of such costs may include a range of relevant resource use, such as staff time (salary 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.

Potential Burdens and Economic Impacts Important to Healthcare Stakeholders

- **Cost of treatment/intervention**
  - Staff time
  - Costs of medication
  - Changes in medication dosage
- **Utilization:** Without associated costs.
- **Costs associated with changes in utilization**
  - Costs of increased, decreased or shifted visits of different types (ED, primary care)
  - Costs of changes in length of stay
  - Shifts in care site (e.g., outpatient versus inpatient)
- **Costs of establishing/implementing new intervention (program costs)**
- **Employer burden:**
  - Absenteeism
  - Reduced productivity from presenteeism

To ensure the data being collected are appropriate and relevant, applicants must 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 have an impact on patient outcomes. One clear example of when collection of economic data may be important is in the 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, this information may prove valuable to decision makers at all levels – from payers, health systems administrators, as well as patients.

Different stakeholders will use different types of analysis, such as return on investment (ROI) to inform their decisions, and they will draw on information regarding patient burden and economic impact in a variety of ways. The amended authorizing law does not direct PCORI to conduct such analyses on behalf of stakeholders. However, PCORI should provide data on burdens and economic impacts that will contribute to the evidence stakeholders have available to conduct analyses to inform their decisions.

Principle #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.

PCORI's foremost mandate, as dictated in our authorizing law, is to support the conduct of comparative clinical effectiveness research. Consistent with the amended language of the authorizing law, PCORI's intention is that funded comparative effectiveness research "shall be designed, as appropriate, to take into account and capture the full range of clinical and patient-centered outcomes," including potential burdens and economic impacts. PCORI will allow studies that include the measurement of the relative costs of care of two or more alternative approaches within the context of CER. <u>However, PCORI does not intend to fund studies for which cost and economic impacts are the primary outcome.</u>

In all research that PCORI funds, PCORI seeks to apply the standard that study outcomes should be relevant and important to patients and other stakeholders. To meet this standard, 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 assure that PCORI-funded research will provide evidence that is ultimately relevant and impactful 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 standard shall apply when considering whether and what cost and other economic impact data a research study should capture. Applicants shall justify their selection of outcomes, including burden and economic impact, and explain when those outcomes are not relevant to the decision being evaluated.

In addition to demonstrating the relevance and importance of data on burdens and economic impact that an applicant proposes to collect in a PCORI-funded study, applicants should also consider the feasibility of capturing these types of data when submitting applications for funding. This includes considering the availability and accessibility of relevant data, the added burdens capturing these data may impose on study participants, ensuring the data are captured from reliable sources, and the potential limitations of the data.

PCORI will consider, when supporting applicants' proposed collection of economic impact and cost data, whether the applicant is leveraging the unique opportunities afforded by their comparative clinical effectiveness research 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.

Principle #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.

The language of the authorizing law as amended charges PCORI with consideration of the full range of "outcomes data" – that is, the collection of data relevant to potential burdens and economic impacts of healthcare interventions. As such, and described in the principles above, PCORI-funded CER can include the capture of data on these impacts.

PCORI understands, however, that to facilitate the consideration of such data, some studies may propose to extend their activities from the simple collection of existing data to the measurement of cost elements and/or to the conduct of limited analysis. For example, a study might collect data on a variety of different cost components that factor into the delivery of an 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 (linens, cleaning), oxygen, cardiac monitoring, etc. 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 limited analysis is consistent with the intention of PCORI's authorizing law. PCORI applicants should ensure their proposed capture of economic impact includes such analyses, as appropriate – and that their report of study findings includes presentation of the results.

There are, however, statutory limits on the types of analyses that PCORI is authorized to fund. In accordance with PCORI's authorizing law as amended by its reauthorizing legislation, prohibitions against PCORI conducting cost-effectiveness analyses and/or developing cost-per-quality-adjusted-life-year thresholds remain in place, as do prohibitions against mandating coverage, reimbursement, or other policies for any public or private payer and prohibitions against research findings that include practice guidelines, coverage recommendations, payment, or policy recommendations. Although PCORI-funded studies will be expected, as appropriate, to collect data on the full range of outcomes, including on the burdens and economic impacts of two of more treatment options, and to conduct limited economic analyses to enhance the usability of these data as inputs to decisions, such analyses must fall well short of cost-effectiveness analysis. In short, such analyses may not aggregate findings on health outcomes with findings on economic impacts – as in a cost-effectiveness ratio. Further, such studies should not be designed in such a way as to inform resource allocation decisions by aggregating and summarizing comparisons between alternative interventions.

Next Steps

PCORI seeks to incorporate feedback from the healthcare community to help inform and shape final principles and guidance for future applicants for research; and will allow applicants to include considerations for the full range of outcomes data as a component to a study consistent with PCORI's authorizing law, as amended by the reauthorizing legislation once final Principles are approved by PCORI's Board of Governors in early 2021. In particular, PCORI seeks input on the following questions, some of which are reflected in the principles as currently described:

1. What are the advantages/disadvantages of a requirement for all PCORI-funded research to capture burden and economic impact data? What are reliable indicators of scenarios/ types of studies where the capture of this data will not contribute to overall importance or value of the research study findings?

2. Given the limits set in PCORI's authorizing statute, it is clear that **data collection** is in scope, and that **cost-effectiveness analysis** is out of scope. Understanding that there are circumstances where additional analysis of data may offer benefit – but that economic analysis should not be a primary goal of PCORI-funded studies – we seek input on the type of analyses that will benefit stakeholders, and the specific uses and advantages of each type of analysis.

- Analysis of specific utilization, e.g. hospital days or emergency care department visits.

- Limited cost analysis to aggregate data on a specific cost component, such as the cost of an outpatient visit for targeted patients.

- Cost of intervention: Cost analysis to aggregate all components of financial investment associated with using an intervention (no offsets included).

- Net cost of intervention: Cost analysis to aggregate the financial investment associated with using an intervention, accounting for offsets based on **observed impacts** (i.e., observed during the course of the study) of the intervention on utilization.

- Impact analysis: Cost analysis accounting for the costs of the intervention, and costs associated with impacts on health care and health outcomes into the future (e.g., 5 years).

Additionally, PCORI understands the complicated nature of conducting research that considers the full range of outcomes data, as appropriate, including those on the potential burdens and economic impacts of the utilization of medical treatments, items, and services on different stakeholders and decision-makers. As directed in PCORI's authorizing law and amended by PCORI's reauthorizing legislation, PCORI's Methodology Committee is tasked with developing and improving "the science and methods of comparative clinical effectiveness research by developing and periodically updating" methodological standards for research.[6] PCORI, leveraging the support and expertise of our Methodology Committee, does intend to undertake efforts to develop "methodological standards" to more fully inform how PCORI-funded studies should consider/capture relevant economic/cost data in the conduct of their research. PCORI ensures that this process will be transparent consistent with the requirements of PCORI's authorizing law.[7]

[6] Social Security Act. Section 1181 [42 U.S.C. 1320e] (d)(6)(C)
[7] PCORI Methodology Standards