

January 31, 2022

Nakela Cook, MD, MPH
Executive Director
Patient-Centered Outcomes Research Institute
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Dear Dr. Cook:

As the Chairman of the Partnership to Improve Patient Care (PIPC), I am writing to comment on PCORI's Proposed Research Agenda. We are pleased that the research agenda places a strong emphasis on improving health equity and addressing social determinants of health. If PCORI is to address research gaps that are imperative to promote health, its research agenda will need to get more specific and its methodologies will need to be more clear.

In our comments on PCORI's National Priorities, we stated our support for PCORI's goals. We encourage PCORI to do direct outreach to organizations representing patients and people with disabilities to get beyond the big picture and to concretely determine what PCORI intends to prioritize and research. This will require tough decisions for PCORI to identify specific diseases and conditions for its focus and health systems changes that require increased attention from researchers. With a 10-year reauthorization, PCORI is in a strong position to demonstrate the value of patient-centered outcomes research by creating a thoughtful research agenda that demonstrates how its research can genuinely move the needle for patients and improve health care.

Our recommendations focus on PIPC's perspectives related to research methods and the need for standards and usable data.

PCORI's new authority to collect economic data is an opportunity to inform the field and drive more investment in understanding outcomes that matter to patients. When PCORI was debated before its creation in 2010, Congress expressed concern that its work could drive a one-size-fits-all approach for health care decision-making, and therefore included safeguards in its statute to ensure its work was not used against patients by restricting coverage. As part of reauthorization, Congress explicitly called on PCORI to collect outcomes data, including economic data on outcomes such as caregiving, while upholding PCORI's protections against using discriminatory metrics such as quality-adjusted life years (QALYs). In the next phase of PCORI's work, PCORI should lead in standardizing patient-centered methodologies for collecting and analyzing outcomes data by engaging with experts with demonstrated expertise in the

conduct of research using nondiscriminatory methods and disseminating its research and standards in a way that is usable by decision-makers and decision-aid developers.

Fund research that fills patient- and stakeholder-prioritized evidence gaps and is representative of diverse patient populations and settings. This piece of PCORI's agenda is highly important because decision-making tools are often inadequate due to a lack of patient outcomes data, from shared decision-making tools to patient-reported outcomes measures. In addition to filling these gaps within PCORI-funded research, PCORI can play an integral and relevant role in establishing methodological standards for the collection of this data. This would allow entities using data to develop decision aids to themselves assess the quality of the data they are using. PCORI's research agenda already highlights some of those standards. Research should be representative of diverse patient populations and settings; consider health equity; leverage the full range of data resources and partnerships; examine the diverse burdens and clinical and economic impacts important to patients and other stakeholders; address health drivers that occur where people live, work, learn, and play, and; communicate evidence so the public can access, understand, and act on research findings. These fundamentals are a good start for advancing standards that are useful for decision-makers and decision-aid developers to assess when data is useful for making a decision versus when it has limited use.

Fund research that aims to achieve health equity and eliminate health and healthcare disparities. Today, data from randomized clinical trials (RCTs) and health utility preference weighting surveys tend to rely on inputs from Caucasian populations. When communities of color are underrepresented in the data that is relied on to determine the effectiveness of a treatment or service, the effectiveness for those populations is likely to be underestimated.¹ PCORI has an opportunity to address this problem by improving the representation of people of color in research, identifying the limitations of data that fail to sufficiently include people of color and improving representation in the underlying data that could potentially improve health utilities. The latter point is very important. While the field of value assessment is moving beyond use of QALYs, more patient-centered methodologies are only as good as the data that is used to inform the underlying health utilities that are weighted to determine how effective a treatment may for a patient or subpopulation of patients. A more equitable health care system will require more targeted information that is not based on averages.

Fund research that builds the evidence base for emerging interventions by leveraging the full range of data resources and partnerships. In addition to federal agency partners, PCORI has the advantage of being a contracting organization, as opposed to making grants, which gives PCORI the ability to provide explicit guidance to researchers about engaging community partners that will bring needed perspectives. When organizations representing patients and people with disabilities, as well as community-based organizations, play a leadership role in research, they are in a better position to hold researchers accountable for meeting standards

¹ <https://www.nmqf.org/nmqf-media/traditional-value-assessment-methods>

for patient-centeredness – centering research on their expertise about outcomes that matter to patients while utilizing the expertise of researchers to achieve rigor. As PCORI considers entities with whom to contract, it is essential that those partners demonstrate the ability and expertise to conduct research using methods that do not discriminate, particularly for identifying and measuring outcomes that matter to patients in the decision-making process that will drive health system improvements.

Fund research that examines the diverse burdens and clinical and economic impacts important to patients and other stakeholders: As with the first research agenda item, this element of PCORI’s research agenda would fill important data gaps. As an example, the statute reauthorizing PCORI called out caregiving as an outcome meriting attention because it represents a significant data gap and an important burden experienced by patients that is not considered as part of payer-centered analyses of costs and benefits. We recommend an analysis of existing PCORI-funded studies to identify the outcomes that engaged patients and other stakeholders sought to be measured as a good start for this process. The diverse burdens and economic impacts that PCORI identifies from its existing studies could then be expanded in a process of engagement with relevant stakeholders – especially patients and people with disabilities – within PCORI’s more specific research agenda so as to be relevant to the disease, condition, population or health system being studied. Throughout this process, PCORI would make available to its funded partners its findings so that those research sponsors have a guide as to what outcomes and burdens are important to collect and analyze.

Fund research that focuses on health promotion and illness prevention by addressing health drivers that occur where people live, work, learn, and play: Social determinants of health are an essential and relevant focus for PCORI’s research agenda. We also agree with PCORI’s proposal to examine the use of and investment in health-promoting resources in the community. While HHS has not advanced its shared decision-making program as authorized in the Affordable Care Act, PCORI has a tremendous opportunity to drive preference sensitive care as referenced in the statute. PCORI could identify the health drivers in communities, fund research that measures the impact of treatments and services on those health drivers and disseminate it in a manner that allows it to be used to inform the development of decision aids in communities that promote health, including shared decision-making and patient-reported outcome measures.

Fund research that integrates implementation science and that advances approaches for communicating evidence so the public can access, understand, and act on research findings. Methods matter. As part of its reauthorization statute, the PCORI Board of Governors was given authority to select members of its Methodology Committee. We strongly recommend PCORI use that authority to select members of the Methodology Committee that have a strong background in patient preference information studies and methods for collecting and analyzing economic data that do not rely on the QALY.

More broadly, it is essential for PCORI to bring into its work experts that do not view QALYs as the “gold standard” and have demonstrated expertise in patient-centered methodologies. PCORI has a tremendous opportunity to be a leader in standardizing what it means to collect and analyze outcomes that matter to patients so that studies are viewed as credible. It has been frustrating for the patient and disability communities that payers, policymakers and developers of decision aids typically view patient preference information and patient-reported outcomes as less rigorous data, despite its value for identifying what patients value for their care in real world settings. When health care decisions are made without reliable data on the experience of patients and their preferences, we have a systematic problem. The solution to that problem is a clear and credible source for decision-makers to not only directly access outcomes data meeting criteria for being patient-centered, but to also better understand when – or not – the outcomes data they are relying on is indeed credible and representative of diverse patient perspectives. Otherwise, decisions continue to be made based on data and research that is unreliable and fails to capture what matters to patients.

As PCORI considers standards for the methods that researchers will use to collect this outcomes data, we want to reiterate our recommendations from our comments on PCORI’s National Priorities:

- Meaningfully **acknowledge diversity and differences among patients and people with disabilities**, avoiding methods that obscure these differences. Summary measures such as the cost-per-quality- adjusted-life-year, which embody a “one-size-fits-all” mentality of value, should not be used. We urge PCORI to avoid any measures for analyzing data that are based on averages and fail to recognize heterogeneity among patients.
- Use **transparent processes and methods** that allow for patients and people with disabilities to understand how the outcomes data was collected and analyzed, including its limitations.
- **Meaningfully engage with patient and provider organizations** to achieve consensus on data to be collected, methods to analyze it and communication of its meaning and limitations.
- **In addition to stakeholder engagement, rely on a range of sound, patient-centered sources of evidence** such as data registries and other patient-centered outcomes research to identify the outcomes to collect and analyze that could also potentially be used as comparators to the data collected in the PCORI-funded project.
- **Address costs and benefits that matter to the patient**, including costs such as lost time at work, risk of disability, and the potential need for caregiving.

Thank you for this opportunity to provide comments.

Sincerely,



Tony Coelho
Chairman
Partnership to Improve Patient Care