

August 27, 2021

Nakela Cook, MD, MPH
Executive Director
Patient-Centered Outcomes Research Institute
1828 L Street NW, Suite 900
Washington, DC 20036

Dear Dr. Cook:

We are writing to comment on PCORI's Proposed National Priorities for Health. As we have said in the past, we believe PCORI has a key role to play in advancing a health care system that delivers high-quality, affordable, and patient-centered care. PCORI's strong emphasis on improving health equity and addressing social determinants of health are elements that we strongly support. As you implement these new priorities, we hope you will consider our insights and recommendations on how PCORI can best translate its priorities into an agenda that meaningfully improves health care decision-making, advances health equity, and supports personalized and precision medicine.

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

We are pleased that PCORI will review existing and emerging innovations with consideration of associated unintended consequences, adverse events, barriers to care, burdens and economic impacts, and widened disparities in care outcomes. We look forward to PCORI using its new authority to collect and analyze cost and economic data as a catalyst for developing standards that will assure the information collected represents methods that are both rigorous and centered on preferences, needs and outcomes that matter to patients and people with disabilities. Doing so will not only improve PCORI's comparative clinical effectiveness studies, but also will drive the field of outcomes-based research to rely on PCORI's standards with assurances that collecting and analyzing information on patient preferences and outcomes can be done in a manner that is reliable, rigorous and useful. We saw this dynamic happen in PCORI's first ten years as PCORI demonstrated that engaging patients and people with disabilities in research served to improve research, not undermine it – other research entities are now putting more emphasis on stakeholder engagement because of PCORI's proof of concept. Similarly, as PCORI sets the standard for how to collect and analyze data representing the real-world experience of patients and people with disabilities, including their burdens, costs, and preferred outcomes, the more we can expect to see investment in this type of patient-level data from other research entities using methods that meet those standards.

As PCORI considers standards for the methods that researchers will use to collect this outcomes data, we would suggest the following:

- 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.

PCORI’s Collection of Patient-Level Data Reflecting Burdens and Preferences is Important to Achieve Health Equity

As you know, it is challenging to compare the clinical effectiveness of treatments without patient-level data to understand patient goals for their health and experiences with treatment. Similarly, this missing information creates challenges for effective shared decision-making between patients and providers, for understanding the value of treatment for specific heterogenous patient subpopulations (which is especially important to achieve health equity) and exacerbates payer-level decisions that impede access to needed care by failing to recognize how patients differ in responding to treatment and in their treatment goals – thus undermining the goals of personalized and precision medicine. Achieving health equity requires recognizing the heterogeneity of people living with chronic conditions, disabilities, and other illnesses. Collecting and analyzing this data with heterogeneity in mind will allow PCORI to drive a more robust understanding of how patient subpopulations differ both in terms of how treatments impact them clinically, but also in terms of their differing preferences, costs and burdens related to their condition and differing social determinants of health.

Patients and People with Disabilities Continue to Support Statutory Protections Against Discrimination

PIPC strongly supports PCORI’s mandate of conducting research on the comparative clinical effectiveness of medical treatments and services, as well as the statutory prohibition against cost- effectiveness analysis. At the same time, the new language in PCORI’s reauthorizing legislation on cost-related outcomes provides the Institute with new potential opportunities.

Today, we are pleased that Section 1182 of PCORI's statute had the impact intended by Congress to ensure that PCORI-funded research did not discriminate and was not misused in Medicare coverage decisions or to prevent individuals from making health decisions consistent with their personal values, which were issues of significant controversy in its creation. Ultimately, PCORI was created because Congress was able to provide reassurance to those on both sides of the aisle that PCORI would be impactful in improving our health care system, without undermining the clinical knowledge of providers and the values by which patients make decisions. Within PCORI's national priorities, we urge PCORI to remain vigilant in protecting patients and people with disabilities from discrimination by avoiding use of discriminatory metrics and clarifying the differences among treatments and services for subpopulations.

Specificity Allows for More Robust Stakeholder Engagement and Focus on Questions Important to Decision-Making

When PCORI was first established, we urged it to establish a formal research priority-setting process to establish an explicit national research agenda. We continue to believe that this represents a critical aspect of PCORI's work to ensure it is responding to questions of greatest importance to our health system, including studies comparing outcomes for drugs and devices, and to provide a catalyst for the broader research community. We appreciate that PCORI is updating its priorities but would urge PCORI to identify areas of research more specifically for public comment that can then be translated into a research agenda consisting of specific research questions. Broad national priorities are important, but specificity would allow for more robust stakeholder engagement and assurance that PCORI is focusing its research on topics that genuinely represent issues that patients and people with disabilities most care about.¹

Conclusion

In conclusion, we urge PCORI to work closely with stakeholders in identifying the topics to be prioritized within each broad area, the research agenda for each topic, and the outcomes data to be collected and analyzed as part of the research. We look forward to the next era of PCORI's work as the institute standardizes the methods for collecting and analyzing outcomes data, including cost and economic data, thereby building the evidence base reflecting outcomes that matter to patients, including their preferences and priorities, and capturing the heterogeneity among patient subpopulations within the evidence. Please reach out to Sara van Geertruyden at sara@pipcpatients.org with any questions or concerns.

¹ <http://www.pipcpatients.org/resources/white-paper-road-map-for-prioritizing-research>

Sincerely,



Tony Coelho
Chairman
Partnership to Improve Patient Care