

May 1, 2020

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Dear Dr. Cook:

We want to welcome you to your new position as Executive Director of the Patient-Centered Outcomes Research Institute (PCORI). As you know, the Partnership to Improve Patient Care (PIPC) was pleased to support Congress' reauthorization of PCORI for 10 years and is excited to engage with you, your staff, and the Board of Governors in planning for 10 more years of leadership in the conduct of patient-centered outcomes research (PCOR). We believe PCORI has a key role to play in advancing a health care system that delivers high-quality, affordable, and patient-centered care, and we hope you will consider our insights and recommendations to PCORI for building on its first 10 years.

As a coalition, we came together over 10 years ago for the purpose of supporting Congressional action to establish an institute to conduct comparative clinical effectiveness research that is centered on building an informed health system for patients, people with disabilities, and their providers that improves health decisions. We advocated strongly for the many provisions in PCORI's statute that give a voice – and a vote – to patients in the process of conducting research. We also sought to ensure that the statute creating PCORI included certain protections for patients and people with disabilities against traditional methods for conducting research that discriminate against people with disabilities, serious chronic conditions, seniors, ethnic minorities and others that do not fit the average.

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.

We recognize that patients and people with disabilities remain concerned about whether they can afford the tests and treatments they need. As I stated in 2010, comparative effectiveness research can form the foundation for meeting “the critically important challenge of controlling health care costs while avoiding oversimplified rationing of patient care.”¹ The U.S. has advanced several policies supporting personalized and precision medicine, explicitly calling on policymakers to support the creation of tools that support individualized health care decisions consistent with patient values using patient decision aids and shared decision-making.² The information generated by PCORI is essential to this kind of informed health system that will contain costs by driving the right care to the right patient at the right time, thereby avoiding unnecessary care and costly adverse events. Now more than ever, we need solutions that are both evidence-based and patient-centered.

This is a crucial moment to build on PCORI’s success for another 10 years. Success, we believe, will entail effectively connecting PCORI and its research strengths to the current, pressing needs of our health care system and the patients and caregivers it serves. We are framing our recommendations around three principles that have been important to us since the creation of PCORI: relevance, timeliness, and trust. Relevance involves research to address the questions that matter most; timeliness requires delivering results when and where they are needed by decision-makers; and trust involves both procedures (e.g., that are fully transparent and fully engage patients, caregivers and people with disabilities) and standards (e.g., delivering objective scientific results vs. subjective value judgments). All of these principles must, of course, be in the service of PCORI’s mission to conduct outcomes research that is patient-centered.

Revisions to PCORI’s mandate made during its reauthorization also raise important issues to consider, in particular the changes in the make-up of the Board of Governors, oversight of the Methodology Committee, and clarification of the ranges of outcomes that can be included within PCORI’s mandate to conduct patient-centered outcomes research. With these considerations in mind, we are pleased to offer the priority recommendations below:

- **Create a national agenda for research priorities:** 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 would be pleased to work with PCORI staff in creating a more formal priority-setting process.³

¹ <https://www.healthaffairs.org/doi/full/10.1377/hlthaff.2010.0632>

² Section 3506 of the Affordable Care Act

³ <http://www.pipcpatients.org/resources/white-paper-road-map-for-prioritizing-research>

- **Expand collection of and access to patient-centered outcomes:** 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. We discuss this potential in more detail below.
- **Advance use of patient-centered outcomes in value assessment:** While PCORI’s mandate does not include economic value assessment, we do believe the Institute could play an important role in enhancing this field. With the growing interest in value-based health care and value assessment, PCORI is uniquely positioned to improve value assessment by providing more robust data on patient-centered outcomes for use by assessment organizations.
- **Promote patient-centered methods:** With the shift to the Board of Governors selecting the Methodology Committee, we encourage prioritization of applications submitted by individuals with experience in patient-centered research, data collection and implementation, especially patient and disability representatives. With more direct oversight of the Methodology Committee, PCORI also has an opportunity to ensure it is prioritizing key methods gaps, such as consideration of patient heterogeneity.

New Research Priorities:

We strongly supported the addition of intellectual and developmental disabilities and maternal mortality as research priorities. People with disabilities are particularly excited to be part of a process of determining research priorities and a research agenda for intellectual and developmental disabilities, an area that has traditionally lacked significant attention from researchers. We encourage PCORI to formally engage early with stakeholders, especially patients and people with disabilities, in their efforts to establish an appropriate research agenda for each new topic. We recommend using the priority-setting roadmap and process for setting research agendas in these areas. As we recognized in that white paper, one of the challenges PCORI will face in the creation of new advisory panels is ensuring they fulfill their purpose of eliciting the input of patients, clinical experts, and practicing physicians with knowledge and expertise in various diseases and medical specialties. This will be particularly important in the ranking of research priorities. It will be useful for PCORI to engage with the patient and disability communities representing these new research priority areas for recommendations related to clinical experts and practicing physicians that would be effective participants on related advisory panels.

Consideration of the Full Range of Outcomes Data:

A new provision was also added to PCOR’s statute to consider the “full range of outcomes data” to 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.”

Since our work in support of the creation of PCORI, PIPC has strongly advocated for researchers to measure outcomes that matter to patients in making healthcare decisions, as opposed to driving conclusions based on flawed standards that ignore outcomes that matter to patients, overlook important differences between patients, or discriminate against particular patient groups such as the elderly or people with disabilities. As previously noted, we strongly support the protections included in the Affordable Care Act that focus PCORI on comparative clinical effectiveness and health outcomes while precluding cost-effectiveness analysis. This protection has been essential to building patient trust and advancing a commitment to patient-centeredness.

As a result, PCORI must strike a careful balance in conducting research within these key parameters, while at the same time considering the types of economic outcomes identified in the Institute’s reauthorization. The collection of out-of-pocket cost and future cost data must not conflate clinical and cost effectiveness. In the real world, patients want information on a treatment’s impact in terms of the outcomes they are trying to achieve for their health and quality of life, and will compare those treatments based on their own personal and financial costs associated with treatment (which may differ based on how they are insured).

We believe an appropriate starting point could be to ensure that any cost outcomes that are considered are those that are patient-centered (i.e., those most relevant to patients). Some of these potential “patient-centered cost outcomes” are identified in statute, such as out-of-pocket costs or caregiver burden. As PCORI considers implementation of this section of statute, we urge it to fully engage representatives of patients, caregivers, and people with disabilities. It is also important to implement this policy in a manner that does not contradict PCORI’s existing statutory definitions of research and patient protections.

That said, there are aspects of PCORI’s new statutory mandate regarding patient-centered outcomes data that are promising and could lead to significant contributions to patient-centered outcomes research. For example, PCORI may have significant opportunities to collect data related to caregiving, productivity, symptoms and side effects, how treatments are administered, and other concerns for patients through mechanisms such as clinical and patient registries that further involvement of patients in research. Regardless of the outcome, it is important that PCORI adhere to its mission of patient-centeredness and ensuring that patients

are engaged throughout the process, and that the process is fully transparent to all stakeholders. In doing so, it is essential that patients and people with disabilities inform the data to be collected, how it will be used in the research, and how it will be communicated as an indicator of an intervention's effectiveness for each subpopulation. Additional considerations include where the data is being housed and whether it will be made publicly available. It is important that these standards are developed in collaboration with the patient community and there is an opportunity for input from all stakeholders before finalization.

Ultimately, PCORI will be responsible for advising its contracted researchers on how to collect this data. Therefore, we urge PCORI to create a new advisory committee of patients, people with disabilities, and others engaged in innovative patient-centered research methodologies to develop this guidance to researchers. The advisory committee should also assist in updating PCORI's patient engagement rubric to reflect the need for input from patients related to collecting this outcomes data and its use. Researchers will then be better equipped to capture potential burdens and economic impacts that reflect patient priorities for care and treatment.

Elevating Patient-Centeredness in Value Assessment

As noted above, PCORI's statute clearly precludes it from conducting cost-effectiveness analysis or value assessment. At the same time, particularly in view of the growing movement towards value-based health care and interest in value assessment, we believe PCORI has significant opportunity to foster greater patient-centeredness by organizations conducting value assessment. As recently noted by academics with expertise in the field, one challenge frequently encountered by those conducting value assessment is the ability to identify and capture the full range of disease-specific treatment benefits that are meaningful from the patient perspective.⁴ PCORI's mission would closely align with efforts to identify and make public disease-specific patient-centered outcomes. We also look forward to learning from PCORI's work with the Innovation and Value Initiative related to value assessment methods and solutions.⁵

Transfer of Responsibility for Appointments to the Methodology Committee:

As part of reauthorization, Congress called on the PCORI Board of Governors, as opposed to the GAO, to select members of the Methodology Committee. This is an excellent opportunity to ensure inclusion of nontraditional experts that can support PCORI's mission to do research differently. In particular, we urge the Board to recognize that the Methodology Committee should include representatives with an expertise in patient centered research, data collection and implementation. It will be useful for the Board to select Methodology Committee

⁴ <https://link.springer.com/article/10.1007/s40258-019-00525-z>

⁵ <https://www.pcori.org/research-results/2019/defining-needs-and-progress-toward-improving-methods-value-assessment>

representatives that can be responsive to the needs of their contracted researchers and PCORI's research agenda.

As you know, we are currently in a time of significant evolution in the methods used for comparative clinical effectiveness research. PCORI's own efforts to build the Patient-Centered Outcomes Research Network (PCORnet) is part of this evolution, reflecting increasing demand for the patient-centered data that drives research. In fact, new legislative provisions encouraging PCORI to collect additional outcomes data would require a Methodology Committee that has a unique background and expertise in the collection of patient-centered data, as opposed to the more traditional research methodologists whose expertise may be limited to the conduct of randomized clinical trials (RCTs) or other traditional methods. For example, registries are a useful tool for collecting patient-centered data related to caregiving, productivity, symptoms and side effects, how treatments are administered, and other concerns determined by patients to be outcomes that matter. Methodology committee members should be representative of the innovative patient-centered research methods that PCORI was created to advance.

Conclusion

It is vital to preserve the fundamental strengths of PCORI that have fostered strong buy-in and trust from the patient community over the last 10 years. PCORI's patient-centered mission is driven by a balanced Board of Governors that includes patient and physician representatives, a focus on comparative clinical and patient health outcomes rather than cost effectiveness, and an open, inclusive process that engages patients in a meaningful way. As discussed, we strongly support the essential safeguards in Section 1182 of the existing statute that protect patients by prohibiting PCORI from conducting studies that discriminate using cost-effectiveness thresholds, and by prohibiting Medicare from referencing such studies in making decisions about coverage, reimbursement and incentive programs.

By creating and reauthorizing PCORI, Congress committed to build the evidence base for improved health decisions, seeking to empower patients and drive innovation and value in health care. PCORI has an opportunity to implement this vision by building on its work to change the culture of research to be more patient-centered and ensure that its work meets the needs of an evolving health care system. We are excited to be engaged partners with you in this important work.

Sincerely,





Tony Coelho
Chairman, Partnership to Improve Patient Care