November 1, 2019

Dear Senators Warner, Cassidy, Van Hollen, and Capito:

The Partnership to Improve Patient Care (PIPC) is grateful for your work to advance reauthorization of the Patient Centered Outcomes Research Institute (PCORI). I am writing to provide our feedback on the recent discussion draft of the Patient Centered Outcomes Research Institute Reauthorization Act of 2019. 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 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.” Now more than ever,
we need solutions that are both evidence-based and patient-centered. The next 10 years of PCORI will be essential as the institute focuses on dissemination and implementation of its work.

Therefore, we applaud your focus on reauthorizing and strengthening PCORI to address the needs of patients and consumers. PCORI offers a far superior approach to the policies of other countries that authorize the government to judge the value of treatments using one-size-fits-all comparative or cost-effectiveness standards, in direct contrast to Section 1182 of PCORI’s statute. 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.1 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.

This is a crucial moment for Congress to build on PCORI’s success by continuing its patient-centered mission. We support efforts to focus PCORI’s work on ensuring its research is timely and responsive to the needs of those making decisions about new drugs and other treatment options, and that its research findings are more readily available to decision-makers. We are also aligned with the coalition of Friends of PCORI Reauthorization in their advocacy for a 10-year, long-term, fully funded reauthorization of PCORI’s directive to conduct comparative clinical effectiveness research and that maintains PCORI’s patient-centered mission.

Thank you for this opportunity to provide input on the policy changes that are proposed in the discussion draft that have potential implications for PCORI’s current patient-centered mission that we so strongly support, summarized as follows:

- **Board Composition**: PIPC opposes adding more payers to the PCORI Board of Governors, which we believe would disrupt the balance of voices at the table and potentially undermine the voices of our patient representatives.
- **Methodology Committee Selection**: In the context of the larger discussion draft that would add seats to the Board for payers, we are unable to support this proposed policy change to shift the selection of the Methodology Committee to the Board of Governors.
- **Outcomes Data**: We strongly support the current definitions of “comparative clinical effectiveness research” and “research” in PCORI’s existing statute, as well as Section 1182 of PCORI’s existing statute, and request that you modify this proposed policy to ensure it does not contradict PCORI’s existing statutory definitions of research and patient protections.

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1 Section 3506 of the Affordable Care Act
• **Expert Advisory Panel for High-Impact Research:** We support the establishment of an advisory panel for this purpose, whether required by statute or within PCORI’s existing authority.

• **Long-Term Reauthorization and Extension of Funding:** PIPC supports a long-term, fully funded reauthorization that protects PCORI’s patient-centered mission and extends all of the existing funding mechanisms.

• **Continue and Enhance Provisions that Protect PCORI’s Patient-Centered Mission:** We would urge you to consider extending the protections in Section 1182 more broadly to cover Medicaid, the CMS Innovation Center, and other reforms that would apply value judgments to national policy decisions.

**Board Composition:**

PIPC opposes adding more payers to the Board, which would risk pulling PCORI away from the patient-centered work that patients have worked hard to establish over the last 10 years. When PCORI was created, we applauded that patients got not just a voice, but a vote in its work. The current statute provides patients and consumers with 3 seats on the Board of Governors, consistent with the number of seats provided to payers and manufacturers. Over the years, the patient representatives on the Board have worked very hard to ensure that the Board’s decisions are consistent with its patient-centered mission. This work can be seen in PCORI’s evolution from largely funding investigator-initiated research to now funding targeted research developed in consultation with stakeholders, including patients. PCORI’s patient engagement activities are now the model for the broader research community and has played a significant role in changing culture of research to be patient and person-centered. We cannot support adding four seats to the Board of Governors for payers (for a total of 7 payer seats), which we believe would disrupt the balance of voices at the table and potentially undermine the voices of our patient representatives.

**Transfer of Responsibility for Appointments to the Methodology Committee:**

PIPC has long had concerns that PCORI’s Methodology Committee does not include representatives of patients and people with disabilities as is required for PCORI’s Board of Governors. While we applaud the goal of ensuring that the Methodology Committee includes representatives that can be responsive to the priorities and agenda set by the PCORI Board of Governors, we are concerned that the current discussion draft would increase Board representation for payers, and thereby give payers significant power in choosing PCORI’s Methodology Committee. 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, the discussion draft’s provisions encouraging PCORI to consider additional outcomes data would
require a Methodology Committee that has 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 outcomes data related to caregiving, productivity, symptoms and side effects, how treatments are administered, and other concerns for patients that are consistent with PCORI’s existing definitions. Therefore, in the context of the larger discussion draft, we are unable to support this proposed policy change.

Consideration of the Full Range of Outcomes Data:

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 about a treatment’s clinical value based on cost. As noted in my 2010 commentary in *Health Affairs*, “The use of an implicit cost-effectiveness threshold by the National Institute for Health and Clinical Excellence in England and Wales has led to denial of coverage for some new treatments and has provoked controversy among patients and providers. One important difference between the US and UK institutes, of course, is that the Affordable Care Act limits the new US institute’s focus to comparative clinical effectiveness, putting assessment of cost-effectiveness by the institute out of bounds.” This protection has been essential to building patient trust and advancing a commitment to patient-centeredness.

Therefore, we strongly support the current definitions of “comparative clinical effectiveness research” and “research” in PCORI’s existing statute, as well as Section 1182 of PCORI’s existing statute, which together ensure PCORI is focused on outcomes that matter to patients. The collection of cost data has the potential to conflate clinical and cost effectiveness, which are very different. 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 out-of-pocket costs associated with treatment which will differ based on how they are insured. Therefore, we urge you to modify this proposed policy to ensure it does not contradict PCORI’s existing statutory definitions of research and patient protections by replacing data related to costs with patient-centered outcomes data consistent with the statute such as caregiving, productivity, symptoms and side effects, how treatments are administered, and other concerns for patients, or alternatively focusing it more narrowly on the patient impact of payer-level interventions (e.g., formulary and benefit designs) in which cost is an intrinsic component of the intervention. Alternatively, HHS’ Agency for Healthcare Research and Quality conducts cost-related research through various programs such as its Centers for Education and Research on Therapeutics (CERTs) and Healthcare Cost and Utilization Project, which could more explicitly extend from PCORI’s comparative clinical research.
We look forward to further discussion about your goals for this provision.

**Establishment of Expert Advisory Panel for High-Impact Research:**

PIPC appreciates the thoughtfulness behind this policy proposal. As you may know, we have long advocated for PCORI to establish a set of national research priorities that focus its research on high-impact disease areas and treatments. We support the establishment of an advisory panel for this purpose, whether required by statute or within PCORI’s existing authority.

**Long-Term Reauthorization and Extension of Funding:**

PIPC is grateful for your leadership to advance a fully funded 10-year reauthorization of PCORI. As discussed above, we strongly support extending PCORI’s patient-centered mission. As you know, research takes time and resources. Patient-centered outcomes research as conducted by PCORI also requires attention to the infrastructure that makes it possible, including data, engagement and dissemination infrastructures that ensure research translates into improved health care decision-making. A long-term, fully funded reauthorization that protects PCORI’s patient-centered mission and extends all of the existing funding mechanisms will ensure that research doesn’t begin in a vacuum and end sitting on a shelf, but instead is built and designed with patients and other stakeholders to ensure its usability, and effectively disseminated to appropriate decision-makers for actual use.

**Continue and Enhance Provisions that Protect PCORI’s Patient-Centered Mission:**

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. As discussed above, we believe that 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, not 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. We urge you to preserve these safeguards and to even consider extending them more broadly to cover Medicaid, the CMS Innovation Center, and other reforms that would apply value judgments to national policy decisions.

In conclusion, by creating PCORI, Congress committed to build the evidence base for improved health decisions, seeking to empower patients and drive innovation and value in health care. Reauthorization is an opportunity for Congress to further drive patient engagement in PCORI’s research and ensure that it meets the needs of an evolving health care system.
We appreciate your continuous efforts to keep PCORI reauthorization at the forefront of the Senate’s agenda and recognize the value of your work and leadership in getting to a final reauthorization. Please let us know how we can be of further assistance in ensuring that PCORI achieves a full reauthorization to continue its patient-centered mission.

Sincerely,

Tony Coelho
Chairman, Partnership to Improve Patient Care