

**Comments to the Patient-Centered Outcomes Research Institute  
On the Working Definition of Patient-Centered Outcomes Research  
August 31, 2011**

**Does the definition place appropriate emphasis on, and convey the importance of, the “patient-centeredness” of the PCORI mission?**

PIPC is very supportive of language in the working definition that emphasizes the need for research to focus on questions that matter to patients and their physicians. We also are pleased that it recognizes the importance of considering individual preferences, autonomy and needs, and a wide variety of settings and diversity of participants. At the same time, PIPC is concerned about specific elements of the draft definition. Our recommendations to the definition, as well as changes in the process of finalizing it, are provided in more detail below.

PIPC appreciates the efforts of PCORI to ensure that its work is “patient-centered.” This focus on patients has been a defining feature of the debate over comparative effectiveness research in recent years, and was essential to the patient community’s broad support for the provisions of the Affordable Care Act that created the Institute. As noted by HHS Secretary Kathleen Sebelius in 2009, the goal of the research “is to empower patients and providers with the best information on protocols, procedures, and other relevant issues...” This same point was reiterated in a letter from over 60 patient groups to Congress that called on comparative effectiveness to, “Ensure that research supports providers in delivering the best possible care to their patients” and to “maintain a focus on patient and provider needs...” PIPC strongly supports PCORI’s focus on the needs of patients and their caregivers.

The language in the draft definition is not clear enough in conveying this essential focus on the information needs of patients and their caregivers. We are very concerned that the first, most important sentence in the definition of patient-centered outcomes research (PCOR) does not include the word “patient.” The current working definition describes PCOR as oriented towards helping “people” in general, rather than clearly focusing on patients. This makes the research definition much more diffuse, and causes it to lose the focus on patients. PIPC strongly recommends that PCORI replace the word “people” with “patients and caregivers.”

In addition, while PIPC appreciates the recognition that patient and provider voices need to be heard in PCOR, we believe this is not sufficient to achieve true patient-centeredness in research. Rather, PCOR should be focused on providing information that is relevant to patient and provider decision-making and responsive to their needs. To achieve this, PCOR procedures should place primary emphasis on the perspectives and input of patients and caregivers and their representative organizations throughout the research process. The existing framework positions patients solely as the recipients of health care information and does not empower them to play a key role in all aspects of the research cycle. PCORI should revise its research definition to reflect this broader emphasis on and bias towards the needs of patients and caregivers.

PIPC Chairman Tony Coelho stressed the importance of full patient engagement in an October 2010 article in *Health Affairs*, in which he said the central role given to patient input and guidance in the Affordable Care Act “will give patients a direct, meaningful role in setting research priorities,

overseeing the conduct of research, ensuring strong methodological standards, and release research findings,” and “may be the most critical for achieving patient-centered comparative effectiveness research.”

At the most recent PCORI Board meeting in Washington, DC, Kathleen Teixeira, a member of PIPC’s Steering Committee and the Senior Director of Government Affairs for the American Gastroenterological Association, stated that PCOR should be defined as research that is “intended to help patients make informed healthcare decisions” and is “responsive to the expressed needs and preferences of patients and their care providers.”

The statute creating PCORI also identifies attributes of patient-centered outcomes research, such as meeting “patient needs, outcomes and preferences,” as well as having “relevance to patients and clinicians making informed health decisions.” Changing the definition as we propose would capture the stated goals of the statute.

### **Is the definition consistent with the statute that created PCORI?**

The statute creating PCORI has a stated purpose and a consistent focus on clinical comparative effectiveness and patient health outcomes. PIPC is concerned about language in the draft working definition that includes consideration of “burden to individuals, resources and other stakeholder perspectives,” which would expand PCORI’s mandate to include cost-based research. This language should be dropped in order to align the definition with PCORI’s research mandate as defined in the Affordable Care Act, which calls for the Institute to examine comparative clinical effectiveness and health outcomes. It is important to note that the statute only refers to cost considerations in an indirect context as one of many factors that can be considered in deciding which types of interventions should be subject to comparative clinical and health outcomes research. The scope of outcomes to be considered is clearly limited to comparative clinical and health outcomes. Indeed, this focus on health outcomes was essential to the patient community’s broad support for the CER provisions of the Affordable Care Act, in that it provided a strong basis of assurance that the intent of the research was to improve patient care, not to use it to impose cost-based access restrictions.

For example, a January 2009 letter to Congress from over 60 patient groups called on lawmakers to, “Focus CER on comparative clinical benefit, rather than cost-effectiveness. Any legislation should state that funding will be used only to support clinical comparative effectiveness research...”

### **Does the definition adequately convey the rationale outlined in the rationale document?**

PIPC is not sure if the definition adequately conveys the rationale outlined in the rationale document.

The rationale document discusses the process by which the PCORI Board discussed an earlier draft definition provided by the PCORI Methodology Committee, and states “public comments on the draft as well as comments from the PCORI board were incorporated into 8 subsequent hours of deliberation, theme assessment, harmonization, and refinement by a subgroup group.” PIPC provided one of these “public comments” on the initial draft definition options provided by the Methodology Committee. However, PIPC did not receive acknowledgment of receipt of these

comments by the Board or Methodology Committee, and we are unsure whether or how they were considered in the drafting process. We believe acknowledgment of comments and a description of how they were considered are essential elements of any open and transparent process established by PCORI, and ask that the Board include these steps as part of its process of finalizing a definition of PCOR.

### **Additional comments.**

PIPC is concerned that the rationale document describes a drafting process that was led by a subgroup of the Methodology Committee and, as noted above, was not fully open and transparent. PIPC believes that, as with a sound PCOR process itself, the drafting process should be modified to more integrally incorporate the views and input of patients and caregivers during the drafting process.

This could best be achieved by delaying finalization of the definition until such time as PCORI has hired a “chief patient officer” as described during the July Board of Governors’ meeting and creating a patient advisory council as discussed at the same meeting. Lead responsibility for crafting the definition could then be shifted to these individuals. This would ensure that patients, as well as PCORI staff with responsibility for ensuring patient perspectives are reflected in PCORI’s work, have a leading role in defining “patient-centered outcomes research.”

We believe this delay would not impact PCORI’s ability to proceed with its important work (and in fact may reduce the risk of unnecessarily delaying PCORI’s work while the definition is finalized). While the Institute’s definition of PCOR will be very important to shaping its work over the longer term, it is not a prerequisite for the steps the Institute must take to implement a research program in the coming year. In particular, defining PCOR is not one of the duties identified in statute for the Methodology Committee, and therefore is not essential to enable the Methodology Committee to complete its work. In addition, the statute provides a definition for PCORI’s research, basic operating standards, and criteria for priority-setting, thereby giving the Institute the basic foundation it needs to continue building infrastructure and foundation for its research program in the coming months.

While PIPC strongly supports PCORI’s focus on the importance of patient-centeredness, we also strongly believe it is more important to get the definition done right – and with maximum participation from the patient, caregiver and provider communities -- than done quickly. Thus, we believe that a process to define PCOR would be more appropriately managed by PCORI staff (including its Chief Patient Officer) and a patient advisory council if established by the Board.

PIPC appreciates this opportunity to comment to PCORI on its draft working definition of PCOR. We believe the definition has several strong points, including the set of patient-focused questions, and recognition of the differences in individual patient's needs and preferences. We urge PCORI to consider revising the definition and the rationale document to better reflect the defining characteristics included in the authorizing statute as we recommend in our answers above. PCORI continues to make progress in establishing a sound, patient-centered program for comparative clinical effectiveness research. Finalizing a strong definition of patient-centered outcomes research would represent another important step forward for the Institute.