

**PIPC Roundtable on PCORI's Evaluation Framework  
Meeting Summary and Recommendations  
March 7, 2014**

**Meeting Summary**

On March 7, 2014, the Partnership to Improve Patient Care (PIPC) convened a group of its Steering Committee members, along with individuals serving on the Patient-Centered Outcomes Research Institute (PCORI) Patient Engagement Advisory Panel (PEAP) and PCORI staff. The purpose of the roundtable was to discuss how PCORI should evaluate its activities against a patient-centered framework using metrics that are most useful to patients. PIPC Chairman Tony Coelho opened the meeting by emphasizing the importance of PCORI having a patient constituency, and the potential for PCORI's evaluation framework to be a tool for organizing patients in support of PCORI's patient-centered research model.

During the introductory period, PIPC Steering members noted their strong support for patient engagement in research, calling the discussion on how to assess the "patient-centeredness" of PCORI's work "long-overdue." It was noted that other large research organizations may engage patients twice per year in a large meeting of patient groups. In contrast, PCORI's Board Chairman, Dr. Gray Norquist is perceived to be distinguishing PCORI by seeking to establish processes for truly engaging patients in setting the direction of its research priorities and agenda. It was also noted that PCORI is the first major research entity to include patient representatives on the Board, and certainly the first to truly focus on patients' research needs, their reactions to the research, and their role in conducting research.

PIPC Chairman Tony Coelho articulated the strong support of patient groups for PCORI's patient-centered directives. He also warned of the lingering opposition to PCORI's patient-centered infrastructure. The patient representatives at the roundtable agreed that they strongly supported conducting comparative clinical effectiveness research directed by patient needs, outcomes and preferences during the Affordable Care Act debate. Therefore, PCORI must be identified by patients, patient groups, caregivers and providers as a critical part of the healthcare system in order to succeed. A strong evaluation framework should validate to patients that PCORI takes patient engagement seriously, and intends to measure its use and its impact both within PCORI, and outside PCORI.

***What Should PCORI's Evaluation Framework be Measuring and for Whom?***

The roundtable began with a discussion premised on the experience of participants measuring the success of patient groups, which, like PCORI, are also non-profit organizations. But first the question was posed to roundtable participants whether PCORI is a mission-related non-profit or research funder? There was agreement that PCORI is both, yet shared concern that PCORI is mixing the measures of those two distinct roles, which is creating complexity and confusion. It was agreed that stakeholders will measure PCORI's success based on its patient-centered mission, at least in the short-term. Therefore, PCORI should disentangle how it measures the success of its mission, versus its research. Though the conversation during the roundtable

focused primarily on PCORI's evaluation of its mission-related activities, the roundtable participants were clear that the mission and the research are both important - an evaluation of PCORI's patient-centered mission was not a substitute for using sound methodological standards in its research to produce the highest quality studies.

Next, the roundtable discussed the inputs and outputs for potential metrics of success. An analogy was presented of the New York Police Department seeking to lower crime, but measuring numbers of arrests and reported crimes, which were inputs that did not necessarily indicate the output of lower crime. Since the outcome the city wanted to achieve was lower crime, the police became accountable for lower crime rates as opposed to counting inputs such as numbers of arrests and crime reports. And as a result, crime went down.

Non-profits often find themselves measuring inputs that are easy to measure, rather than the outcomes they trying to achieve. Mr. Jim Collins, who wrote *Good to Great* and then *Great by Choice*, was quoted as saying, "Because your outcomes are inherently not measurable, it doesn't mean you're not disciplined to account for them and figure out ways to measure those outputs."

In a situation with inherently unmeasurable outcomes, the fallback is to look at the quality of the work, the performance, and whether or not it actually has an impact and whether or not it is possible to build a brand around that impact. Because PCORI-funded research may not be completed in time to demonstrate impact prior to its evaluation by the Government Accountability Office (GAO) in 2015, nor in time for near-term Congressional scrutiny and long-term reauthorization in 2019, PCORI should instead establish an evaluation framework focused on how well their activities (i.e., research prioritization, study design, dissemination) have met its patient-centered mission and communicate that success within a timeframe that is responsive to scrutiny by PCORI's stakeholders and funders.

PCORI must therefore have the highest quality product possible – both in its mission-oriented activities and in its research. Roundtable participants agreed that showing the quality (rigor) and impact of research is a longer-term endeavor considering that PCORI's research projects are typically conducted over three years. Yet, the mission-related activities must also be the best, and can be demonstrated as such almost immediately. The Patient and Family Engagement Rubric was an example of a high quality product, yet some were concerned that it must be explicitly communicated to the patient community as demonstrating impact. It was also expressed that PCORI may be doing too much, and instead should be disciplined in doing only those things that will result in the highest quality product that no one else can match, or that no one else is doing. PCORI must also identify when a product is not high quality and impactful, and therefore must be eliminated, despite the investment made in it, so as to shift resources to other activities.

### ***What is PCORI's Brand?***

By evaluating the distinctive impact of PCORI's product, PCORI will develop a brand and a reputation for having a high quality impactful product. Without a brand, PCORI is no different from existing research entities. To develop a brand and to be an enduring organization, there was consensus that PCORI should develop a three-year evaluation framework focused on its mission.

There was also consensus that PCORI must be disciplined to ensure it is developing only the highest quality products consistent with that mission. It was suggested that PCORI have clear boundaries to its work, and therefore allow its energies to be conserved for the development of quality products.

There was an acknowledgement that the development of an evaluation framework related to PCORI's mission could be challenged by those within PCORI who view the organization more as a research organization, and who are therefore viewing evaluation from only that perspective. Yet, developing a brand should emanate primarily from PCORI's mission of patient-centeredness, and PCORI should evaluate its research in that lens as well. In addition to developing an evaluation framework that measures how well PCORI met that patient-centered mission, the meeting participants acknowledged and supported PCORI's work to also develop long-term evaluation metrics for its research that are integral to its dual role as a research organization.

Roundtable participants acknowledged that if PCORI is able to create an evaluation framework that demonstrates its commitment to its mission in the near-term, which results in the development of a high-quality brand, natural advocates and ambassadors will emerge to support its existence. What makes PCORI different is its patient-centeredness and its goal to engage patients throughout the research process. That story will be told, and a brand will be built, with an evaluation framework that captures the elements of "research done differently." It is not enough to just have a brand; stakeholders must be aware of that brand, and the evaluation framework could be a tool for its communication.

### *How do we Measure PCORI's Standing Ovations?*

This led to a discussion of "standing ovations," using the Cleveland Orchestra as an analogy. It is difficult to measure the best orchestra, and Cleveland Orchestra wanted to be great. So it identified outputs that it could be disciplined in measuring, such as how often it received a standing ovation, how often it was invited to play internationally, the extent to which other orchestras copied its style, etc. By measuring these outputs, it developed a brand, and is now considered one of the best in the world, and therefore it will endure. By distinguishing what PCORI is doing that is truly unique, and measuring that, PCORI too will build a brand.

As a mission-related non-profit, the group urged PCORI to use qualitative measures, versus quantitative. It was agreed that patient engagement in research is what makes PCORI unique. There are qualitative measures that PCORI could adopt that will make it accountable immediately to its stakeholders, proving to them that PCORI is meeting its mission of patient engagement. These were described, per the above analogy, as "standing ovations." For example, the Patient and Family Engagement Rubric could become a standing ovation, as could its methodological standards for research, particularly if replicated by others. The creation of an office at the FDA for patient engagement could also be partially attributed to PCORI's work, showing a ripple effect.

In response to concerns that these qualitative measures cannot be attributed 100% to PCORI's actions, it was agreed that nothing could be measured if it had to be solely attributable to one factor. As examples, the American Heart Association holds itself accountable for reducing heart disease, and the American Cancer Society holds itself accountable to reduce cancer rates, despite the recognition that there are multiple factors contributing to those outcomes. In the same way, PCORI impacts the culture of research and technology development to be more patient-centered. Patient groups are counting on PCORI to develop an infrastructure that provides for patient engagement in research that ultimately changes the course of research and emerging treatments to be more patient-centered.

### *How does PCORI Tell its Story?*

The roundtable participants also discussed the process by which PCORI engages with stakeholders, and tells its story. Chairman Coelho discussed how the community of people with disabilities began to understand PCORI's potential for addressing issues they care about during a meeting with PCORI's research directors. PCORI described to the meeting participants the types of research that PCORI was funding that was relevant to them, and as a result, the disabilities groups became very interested in engaging with PCORI, and have since identified research priorities and research partners. Similarly, it was suggested that the evaluation framework quantify the number of connections being made to stakeholders, and its impact. There was an acknowledged shift, perceived as valuable, from PCORI's early focus on broad engagement activities to more one-on-one interactions. The roundtable participants urged more of this type of individualized engagement with patient groups as a means to better communicate the value of PCORI's work on specific patient populations so that they are invested in PCORI's success.

The final part of the conversation focused on the need for PCORI to embrace a patient-centered three-year evaluation framework in order to succeed in its mission. Done correctly, it could be a tool for PCORI to tell its story to stakeholders, especially the patients who want PCORI to succeed the most. It will have to be led by PCORI's executive leadership, and these patient-centered measures will need to be incorporated into all that PCORI does to maximize its usefulness in telling PCORI's story.

### **PIPC's Consensus Recommendations:**

- PCORI should develop an evaluation framework that is useful to patients, patient groups, caregivers and providers. Therefore, a mission-focused evaluation framework should focus on PCORI's mission of patient-centered research and patient engagement.
- This mission-related evaluation framework should be developed to show outcomes over the next 3 years. PCORI should also have a separate plan for long-term evaluation of the rigor and impact of PCORI's research. Roundtable participants agreed there are several timelines to consider. First, the GAO is to evaluate PCORI in 2015. Second, PCORI will not have research to disseminate until around 2017. Third, PCORI must be reauthorized by Congress in 2019. Therefore, PCORI needs a 3-year timeline for demonstrating positive results. There could be a bifurcated process to evaluate PCORI's mission over 3

years, and then to have a longer 10-year plan for the impact of PCORI-funded research once research findings are available.

- PCORI must develop a high quality product, which will require a disciplined approach to its engagement practices and programs. It was suggested that PCORI reach out to patients, patient groups, caregivers and providers, especially those with experience in mission-oriented non-profits, to assess the value of activities under consideration so that only those of the highest potential value are pursued.
- PCORI should individually engage with stakeholders such as patient groups to identify to them the PCORI-funded projects that impact their specific patient populations, and thereby get them invested early in the value of PCORI.
- PCORI must be responsive when its programs are determined to not have the outcome intended. For example, PIPC’s members are pleased to see PCORI shifting more funding to the more patient-centered targeted funding announcement process utilizing advisory panels.
- PCORI should embrace the brand “research done differently” so that it is communicated and understood and embraced by patients, patient groups, caregivers and providers. An important step in developing this brand is to better define, through its evaluation metrics, what makes PCORI truly different from other research organizations. This includes PCORI’s goals of patient engagement, the use of advisory panels to prioritize research, and the application of patient-centered criteria that must be included in all PCORI-funded research. This will give PCORI’s brand of “research done differently” credibility.
- To demonstrate outcomes from a 3-year evaluation, PCORI must use both qualitative and quantitative metrics in its evaluation framework. If “research done differently” is the brand, it is the qualitative measures that will immediately distinguish PCORI from other research organizations. Although it is vital for PCORI to conduct rigorous research to ensure it has a high quality product that leads to an enduring high quality brand, it is not the rigor of the research that will immediately distinguish PCORI from others. It is the mission of patient engagement in research that makes PCORI different. Examples of qualitative measures, i.e. standing ovations, were identified by the roundtable participants as:
  - the use and replication of PCORI’s engagement practices and patient-centered programs (e.g. the Patient and Family Engagement Rubric and PCORI methodological standards);
  - increased focus on patient engagement in the operations of other healthcare entities, such as the recruitment and hiring of PCORI’s Chief Officer for Engagement, Dr. Anne Beal by a large pharmaceutical company (an indication that drug companies are seeking to engage patients in research and product development) and the FDA creating its own patient engagement office;
  - assessment of usefulness of research for health care decision-making by patients early in the research process;
  - use of engagement officers and impact on meaningful patient engagement in PCORI-funded research projects;
  - perspectives of engaged stakeholders and researchers toward engagement captured from surveys (surveys could be required by PCORI in their contracts);

- invitations to present PCORI-funded research to patients and patient groups (as opposed to presenting to other researchers);
- increased awards by PCORI to patient organizations and patient-led research projects;
- improvements in the patient-centeredness of funded applications over time, particularly improvements demonstrated by the targeted funding announcement process;
- improvements from the first broad program funding announcements to the last, such as after changing the patient-centeredness criteria and incorporating the Patient and Family Engagement Rubric into the PFA's themselves;
- increasing levels of patient engagement at the front end of research development;
- increased capacity for patients and patient groups to engage, perhaps by showing how patient groups, after engaging with PCORI, are building into their organizations a larger role for patient engagement in research.