



September 14, 2012

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Dear Drs. Gabriel and Selby:

I am writing to you on behalf of the Partnership to Improve Patient Care (PIPC) in response to the request for comments on the draft report of the Patient-Centered Outcomes Research Institute (PCORI) Methodology Committee presented on July 23, 2012, and revised thereafter. We understand the importance of the methodological standards being developed by PCORI's Methodology Committee, and are especially aware of the statute's direction that the PCORI Board of Governors adopt the methodological standards "developed and updated by the methodology committee as well as other actions deemed necessary to comply with such methodological standards."¹ Once ultimately adopted by the Board, these standards will apply to the contracts for comparative clinical effectiveness research funded by PCORI. Therefore, PIPC is providing to PCORI, both the Board of Governors and its Methodology Committee, the comments below.

Process for Developing the Draft Report

PIPC is concerned that the input and guidance the Methodology Committee received in developing the report did not involve strong and diverse representation from the perspectives of patients, people with disabilities, and their physicians and caregivers. In addition, the report was drafted using non-transparent procedures. As a result, it is not fully aligned with the needs and concerns of many patients and people with disabilities. While we appreciate the opportunity to comment on the draft report (as mandated by statute) we urge the Methodology Committee to more fully implement its mandate to reach out to a broad range of stakeholders in its work on standards for patient-centered outcomes research.

¹ 42 U.S.C. 1301(d)(4)

Scope of Draft Report

PIPC is concerned that the draft report reflects the Methodology Committee’s interpretation of a mandate that extends well beyond that of the statute itself. As a result, the report encompasses several issue areas where rigid scientific standards should not be applied. First and foremost is the issue of how PCORI will set national priorities for research.

This issue has been a high priority for PIPC because it goes to the heart of the Affordable Care Act’s goal of moving the field of comparative clinical effectiveness research towards a new patient-centric model. This critically important activity is not compatible with formal scientific “standards,” however, and we feel it would be a serious mistake for the Methodology Committee and PCORI to seek to apply them to priority-setting.

There certainly are more effective and less effective approaches to engaging patients and providers and determining research priorities that are responsive to our real-world decision-making needs. And we appreciate PCORI’s ongoing outreach and work to identify effective approaches to setting specific research priorities. However, this does not mean that this activity can be reduced to a set of systematic scientific standards. Learning what matters to patients and incorporating it at the center for the research agenda is a complex process that occurs at the intersection of medical care, science and policy, and it likely will require a variety of approaches used together in order to succeed. Essential to the success of this process is not scientific standards, but good decision-making procedures that start with seeking input and ideas from patients and caregivers. We do not see this type of early outreach to the patient community clearly incorporated into the Methodology Committee’s approach. Nor does their approach fully appreciate the important role of practicing physicians and clinical experts in defining a research agenda that responds to the needs of patients and their caregivers. In a set of recommendations provided to PCORI by a roundtable of physician specialty societies and PIPC, PCORI was urged to form expert advisory panels as authorized by law with sufficient representation from clinical experts, caregivers and patients, and to implement a process for defining research priorities that enabled meaningful input by expert advisory panels and clinicians more generally.² We hope the Board of Governors fully considers those recommendations.

In the meantime, PIPC recommends that PCORI direct the Methodology Committee to amend its draft report to focus it squarely on the work mandated by the statute. As you know, the statute directs the Methodology Committee to develop and improve the science and methods of comparative clinical effectiveness research by developing and updating “methodological standards for research” and a “translation table.”³ The Methodology Committee incorrectly interprets this mandate in the following statement:

² See Medical Society Roundtable Summary and Recommendations to PCORI.

³ 42 U.S.C. 1301(d)(6)(C)(i) and (ii)

“We have interpreted this legislation to mean that the PCORI Methodology Committee’s role is to advise PCORI on the best methods for promoting its agenda—in prioritizing research, establishing a specific research project agenda, reviewing research proposals, supporting infrastructure for PCOR, improving the conduct of PCOR, and furthering the implementation of PCOR to help patients receive optimal outcomes.”⁴

This statement is contrary to the statute’s direction that the Methodology Committee develop scientific standards for research. As a result, the report seeks to set scientific standards for policy decisions and processes like research priority-setting and patient engagement.

Establishing research methods that help move the field of comparative clinical effectiveness in a patient-centered direction is an important and challenging task. Rather than defining scientific standards for patient-centered priority setting via a closed Methodology Committee process that does not include representatives for patients or people with disabilities, we urge PCORI to develop a new process for priority setting working in close consultation with patients and patient advocacy organizations. For example, the National Health Council has provided to PCORI a framework for engaging patients that captures the breadth of experience of its members, which includes 50 of the nation’s leading patient advocacy groups. Their work provides a truly patient-centered approach to patient engagement that should not be overlooked.⁵

Over-reliance on Existing CER Models

We appreciate the inclusion in the draft report of elements of patient-centeredness, such as recognizing patient differences (heterogeneity) and patient preferences. We recommend that PCORI and the Methodology Committee give greater attention to these factors in the final report, and in particular describe how they should be incorporated in the range of research methods.

At the same time, we are concerned that the draft report relies too heavily on existing approaches to priority-setting employed by the Agency for Healthcare Research and Quality and the National Institutes of Health. For example,

“The Committee provides a framework and narrative overview of topics related to research prioritization and proposes three standards with supporting recommendations...Elements of this framework have already been used by the Agency for Healthcare Research and Quality (AHRQ)...”

The procedures used by AHRQ for priority-setting are more oriented towards the needs of payers. For example, the statute underlying the agency’s Effective Healthcare Programs directs AHRQ to prioritize research that meets the needs of the Centers for Medicare and Medicaid Services and state

⁴ Public comment draft report of the Patient-Centered Outcomes Research Institute (PCORI) Methodology Committee presented on July 23, 2012, and revised thereafter, at 4.

⁵ See http://www.nationalhealthcouncil.org/NHC_Files/files/NHC-Patient_Perspectives_PCORI.pdf

Medicaid agencies, not patients. Therefore, it falls short of the goal of and requirements for patient-centeredness that the statute requires for PCORI.

In addition, AHRQ does not make a draft list available for comment, and does not provide rationale for topics selected. While AHRQ begins with broad solicitations for topics, which we view as positive, the lack of transparency in decision-making makes it unclear to stakeholders how or whether their input was considered, and thus discourages meaningful ongoing engagement. We would reference the comments made to the PCORI Board in Denver, CO by Dr. Lauren Frey, a member of the Epilepsy Foundation, where she described the epilepsy community's concerns about the lack of full patient and physician participation in AHRQ's study of epilepsy treatments, which then led to a deeply flawed report.⁶ It is imperative that PCORI not repeat this flawed process.

The approaches endorsed by these chapters do not provide a clear description of the central role that patients and caregivers should play throughout the process, and could have the effect of minimizing the input received by patients and providers. While this is not the intent of PCORI, it likely would be an unintended outcome if the draft report is adopted with these recommendations. The recommendations outlined on page 40 of the draft report do not describe a process for establishing priorities that engages patients specifically, but instead they seem to rely on researchers to determine best practices. Although the Methodology Committee's recommendations may be informative to PCORI's patient engagement processes, they should not be included in the Methodology Committee's formal report to the Board that is to be adopted and followed by the Board.

Our concern that the draft report may simply be replicating existing, payer-centered procedures also stems from the statement:

“Using a systematic approach to identify gaps in the existing literature and deficiencies in completed studies should reduce investments in research that are unlikely to help answer important questions, while avoiding potentially unethical patient recruitment into unneeded studies and fostering transparency and accountability in funding prioritization. While the [Methodology] Committee notes that AHRQ has advanced the field [identifying evidence gaps] in the United States significantly with its Future Research Needs Approach.”⁷

Although identifying evidence gaps is a function of prioritization, PCORI's statute also makes it clear that “patient needs, outcomes, and preferences”⁸ are key factors. AHRQ does not have a process that engages patients in a meaningful way, especially for the identification of research needs. And the end result can be a report of research findings that have low value, yet by AHRQ's own disclaimer language, can be used as a basis for reimbursement and coverage policies. We note this

⁶ see <http://improvepatientcare.org/sites/default/files/events/052112%20PCORI.pdf> page 320-325.

⁷ Public comment draft report of the Patient-Centered Outcomes Research Institute (PCORI) Methodology Committee presented on July 23, 2012, and revised thereafter, at 36.

⁸ 42 U.S.C. 1301(d)(1)(A)

example to reinforce the importance of a patient-centered process for establishing priorities and a research agenda that is unique to PCORI and meet's the statute's expectations so that PCORI's research is trusted and credible to facilitate appropriate dissemination and uptake of research findings into practice.

Finally, PIPC questions the reliance on “value of information” (VOI) analysis as a tool for PCORI research priority setting, a technique that also has been supported by AHRQ. As we noted in our comments⁹ on the AHRQ a report entitled “Evaluating the Potential Use of Modeling and Value-of-Information Analysis for Future Research Prioritization Within the Evidence-based Practice Center Program”¹⁰, VOI to date has only been applied in one real-world decision-making process – that used by the U.K. National Institute for Health and Clinical Excellence (NICE) (VOI) as part of its methods for setting priorities. Thus, the draft report appears to embrace an approach to research prioritization that defines research value based on the cost-containment needs of government agencies. As noted in the draft, this approach has been used almost exclusively by the NICE in the United Kingdom. By aligning its agenda and methodologies with that of NICE, PCORI would contradict its mandate to be patient-centered and respond to the information needs of patients and providers.

We note that the report's AHRQ-centered recommendations align with the research interests of members of the Methodology Committee, many of whom work at organizations that receive extensive funding from AHRQ. While these may represent cutting-edge research approaches, it is unclear to us whether these approaches will foster a new patient-centered research program. It also is unclear whether a process that included broader representation for the patient and physician communities would have yielded similar recommendations.

Cost Considerations

The Methodology Report amplifies concerns that PIPC expressed earlier this year about PCORI's policy on considering cost in its research agenda. While we appreciated PCORI's thoughtful response to our prior letter (which seemed to indicate that the Institute's interest in cost was limited to ensuring that it does not invalidate the results of comparative clinical studies), we remain concerned about cost language in PCORI's final research priorities. These concerns are heightened by the language in the Methodology Report, which appears to view cost through the lens of reducing use of (e.g., through delaying or denying patient access to) medical items or services.

For example, the report states, “The Committee's view is that in the context of PCOR, cost, like a number of other social barriers, can be a factor in the effectiveness of a given treatment if it

⁹ see <http://dev.improvepatientcare.org/news-media/pipc-comments-ahrq-draft-engaging-stakeholders-identify-and-prioritize-future-research-ne>

¹⁰ Myers, Evan, M.D., M.P.H.; Sanders, Gillian D. Ph.D; et al. (June 2011) Evaluating the Potential Use of Modeling and Value-of-Information Analysis for Future Research Prioritization Within the Evidence-based Practice Center Program, (prepared by the Duke Evidence-based Practice Center Durham, NC under Contract No. 290-2007-10066-I) (Agency for Healthcare Research and Quality, Rockville, MD) AHRQ Publication No. 11-EHC030-EF.

influences choices made by patients and clinicians...Providers may have incentive to favor more costly treatments under the common belief that ‘more is better’ in healthcare.” PIPC notes that for many patients with serious diseases and conditions (such as cancer, epilepsy, mental health conditions, and HIV/AIDS) more diagnostic and treatment options have been better. CER viewed through a payer-centric lens of cost containment could lead to an agenda focused on restricting choices rather than empowering patients and their caregivers to make the best choices.

As we noted in our June letter to PCORI, PIPC strongly supported the creation of PCORI in large part due to the frustration among patients and providers that many existing CER programs were not designed to answer their questions, and did not include research questions that go beyond benefit and risk to include the full range of outcomes that matter to patients, including quality of life, productivity, and patient-reported outcomes. While issues related to costs, value, and efficiency can impact patient care, the expansion of PCORI’s mandate to include these topics will lead to an agenda that ultimately is payer-centric and cost-based. PCORI should align its research agenda, methods, and processes with the definition for patient centered comparative clinical effectiveness with patient-centered research as defined in statute

The multiple, conflicting statements that have been made by PCORI and the Methodology Committee in regards to cost have resulted in confusion and exacerbated an underlying mistrust that comparative clinical effectiveness research ultimately will be used to cut costs by restricting patient choice. We urge PCORI to address this by deleting statement in the draft methods report that would allow for inclusion of cost considerations as a core element of PCORI’s agenda.

Incomplete Description of the “Trust Problem” Related to CER

The lack of broader representation and engagement of the patient community in development of the draft report also may be reflected in the incomplete description of the “trust problem” in the draft report.¹¹ The report overlooks one of the trust problems that many patients and members of the public have with comparative effectiveness research – patients do not trust that payers have their best interests at heart, and therefore do not trust that research funded by payers (public or government) has their best interests at heart. This was noted, for example, in a section on “public trust” in the report released in 2009 by the Institute of Medicine’s Committee on Comparative Effectiveness Research Prioritization.¹² That is why PCORI was structured as an independent Institute with representation from the patient and physician communities, and why full engagement of these communities through transparent, open processes is so vital to PCORI building trust. If this section of the report is retained, it should address this issue as an element of the “trust problem.”

Process

¹¹ Public comment draft report of the Patient-Centered Outcomes Research Institute (PCORI) Methodology Committee presented on July 23, 2012, and revised thereafter, at 5-6.

¹² Committee on Comparative Effectiveness Research Prioritization, Institute of Medicine, report on “Initial National Priorities for Comparative Effectiveness Research” at 145-146 (2009).

While PCORI has stated that there will be a public comment period on the draft report, PCORI has yet to define a clear, predictable, and deliberate process to receive and consider broad public input. The lack of clarity about how comments will be not only considered, but incorporated into the final report, is amplifying stakeholder concerns that their input will not be meaningful. The serious criticism of the process for consideration of comments from stakeholders on the draft research priorities and research agenda should be a lesson for PCORI on the importance of not only providing opportunities for input, but making that input meaningful.

Therefore, PCORI should clearly delineate how the comments received on the draft Methodology Committee report were aggregated and analyzed, including any segregation or weighting of comments according to stakeholders (i.e., researcher v. non-research comments). In addition, a process for reviewing and updating the methodological standards should be explained. And of course, PCORI should take action to address the concerns of those commenting on the report by incorporating suggested changes where appropriate. Once these steps are taken, PCORI will demonstrate that public input has meaning in their process.

In closing, PIPC appreciates this opportunity to provide comments on the draft Methodology Committee report. We look forward working closely with PCORI in the future, and hope that our comments are helpful to you.

Sincerely,

A handwritten signature in black ink, consisting of a stylized initial 'T' followed by the name 'Coelho' in a cursive script.

Tony Coelho
Chairman