

March 15, 2012

BY ELECTRONIC DELIVERY

Joe V. Selby, M.D., M.P.H.
Executive Director
Patient-Centered Outcomes Research Institute
1701 Pennsylvania Ave. NW
Suite 300
Washington, DC 20006

Re: Patient-Centered Outcomes Research Institute (PCORI) Draft National Priorities and Research Agenda

Dear Dr. Selby:

The Biotechnology Industry Organization (BIO) is pleased to submit the following comments on the Patient-Centered Outcomes Research Institute's (PCORI's) Draft National Priorities and Research Agenda, published on the group's website on January 23, 2012.¹ BIO is the largest trade organization to serve and represent the biotechnology industry in the United States and around the globe. BIO represents more than 1,100 biotechnology companies, academic institutions, state biotechnology centers, and related organizations in the United States. BIO members are involved in the research and development of novel interventions to prevent, treat, and cure diseases through the most advanced science.

BIO supports PCORI's goal of increasing the availability of accurate, scientific evidence to inform clinical decision-making. BIO supported the creation of PCORI to conduct comparative clinical effectiveness research, and we maintain an ongoing desire to see the Institute successfully carry out its statutory mandate. In addition to providing feedback on the National Priorities and Research Agenda, this letter also focuses on the importance of PCORI's adoption of a systematic and transparent public input process.

I. Content Issues

- a. *The lack of specificity in the National Priorities and Research Agenda could undermine PCORI's goal of creating a solid evidence base around specific research questions.*

BIO acknowledges that PCORI must walk a difficult line in creating the National Priorities and the Research Agenda, and appreciates the steps PCORI has taken to engage with and solicit feedback from stakeholders. The research topics PCORI prioritizes must be broad enough to describe a vision for future research, but also must be detailed enough to provide researchers with the guidance needed for the Institute to build a solid and cohesive evidence base around specific research questions.

¹ Patient Centered Outcomes Research Institute. "Draft National Priorities for Research and Research Agenda Version 1." January 23, 2012. Available at: <http://www.pcori.org/assets/PCORI-Draft-National-Priorities-and-Research-Agenda.pdf>

BIO is concerned that while the proposed topics provide a broad vision, they do not provide sufficient detail and guidance for researchers.

According to statute, PCORI's research agenda should detail the specific subject areas to which researchers should devote their time and effort. However, the current drafts lack the proper amount of specificity and guidance. Unless it provides this specificity and guidance, PCORI risks ceding its responsibility to the individual researchers applying for funding, who may dictate, or at least strongly influence, what sorts of questions get studied. As a result, PCORI may undermine its ability to create a body of cohesive studies to support evidence-based decisions. For example, the research performed could result in a scatter-shot of topics, without addressing in depth specific research questions. A single study in one of the proposed research areas is unlikely to provide sufficient evidence to have a meaningful impact on health care decisions. If PCORI does not provide the needed specificity, there is a risk that researchers will simply develop a number of unrelated studies that, when taken in aggregate, provide little new and useful information on how to improve health care delivery and outcomes. By providing a more detailed framework, PCORI can ensure that the appropriate research – with the depth needed to guide future health care decisions – can be performed and disseminated. In ensuring that targeted, focused research with the depth needed to answer specific health care questions, PCORI will be well-positioned to meet its goal of improving patient outcomes.

- b. *PCORI's proposed issue prioritization offers an opportunity to expand the reach of comparative effectiveness research.*

PCORI has an important and difficult responsibility in determining the organization's funding allocations for comparative effectiveness research. Unfortunately, it is not clear how the initial priorities were developed. Without adequate information on the decisions behind these priorities or the justifications for the allocations, it is difficult for stakeholders to provide meaningful feedback on the allocations.

Given the information available, BIO is concerned that PCORI's current funding allocations do not reflect an appropriate prioritization of issue areas. For example, PCORI has allocated the bulk of its funding to support research of "assessment of options for prevention, diagnosis, and treatment" without describing the rationale for that prioritization. We urge PCORI to re-examine its allocation determinations. PCORI has a unique opportunity to distinguish itself from existing comparative effectiveness research entities by focusing on ways to improve health systems and delivery, which is an area with significant gaps in research and information. Yet, PCORI proposes to allocate just 20 percent of funding to "improving healthcare systems" by researching topics such as "coordinating care for complex conditions" and "comparing health system-level approaches to improving access." Poor care coordination – inaccurate or incomplete transfer of information, lack of appropriate follow-up, and poor communication – results in preventable hospital readmissions and increased emergency department visits. More information is needed on best practices to achieve better care coordination and in turn, improve patient outcomes. PCORI can establish itself as a leader in comparative effectiveness research by exploring ways to improve health care systems, which is an area that has been largely ignored by other organizations doing comparative effectiveness research. Therefore, BIO recommends that PCORI increase funding for research into improving healthcare systems to fill this much needed void.

Likewise, the dissemination of new research findings is an important area where PCORI can play a leadership role. BIO asks that PCORI provide additional information on the proposed allocation of funds towards dissemination of research. Again, without more explanation of the resource allocation, it is difficult to determine whether the 10 percent specified in the proposal is sufficient to "address individual differences and barriers to implementation and dissemination." For example, it is unclear what the totality of communication and dissemination funding is in light of the funds being transferred to the Agency for Healthcare Research and Quality (AHRQ) for dissemination. BIO seeks information on what proportion of those funds will go toward dissemination of PCORI-generated comparative effectiveness research, and how those funds will interact with the AHRQ transfer funds.

- c. *PCORI should ensure that the scope of research balances patient outcomes improvement with medical innovation.*

In establishing the National Priorities and Research Agenda, PCORI must seek to establish the proper balance between outcome improvement and medical innovation. It is essential that PCORI recognize that the advances made in medical technology over the past century have revolutionized the practice of medicine, and are responsible for significant improvements in patient health outcomes. Therefore, BIO urges PCORI to ensure that its research and other activities capture the critical balance of improving patient health outcomes while also encouraging medical innovation. In addition, BIO urges PCORI to abide by its statutory duties and refrain from funding research that is designed to address coverage or payment decisions.

II. Procedural Issues

- a. *Simultaneous release of the National Priorities and Research Agenda makes it difficult for stakeholders to provide thoughtful comments.*

Under statute, PCORI is required to release the National Priorities and Research Agenda sequentially and allow for two separate 60-day comment periods. However, in an effort to expedite the distribution of funding, PCORI has instead released both of the documents simultaneously. While BIO commends PCORI for making the effort to distribute funds as efficiently as possible, we are concerned that by releasing both documents simultaneously, PCORI has made it difficult for organizations to provide meaningful input on both documents. BIO previously urged PCORI to release the National Priorities and Research Agenda separately and sequentially, with adequate comment periods, so that stakeholders and affected entities could provide thoughtful and thorough comments on these important issues.

BIO is concerned that soliciting comments on new documents while other comment processes are ongoing creates unnecessary confusion for stakeholders. BIO notes that the comment period for the National Priorities and the Research Agenda ends just ten days after the definition of Patient Centered Outcomes Research was finalized and overlaps with the opportunity to offer feedback on the translation table framework. With PCORI requesting feedback on three important issues simultaneously, stakeholders' ability to spend the time to develop appropriate and thoughtful comments is

severely limited. Due to the interconnectedness of these documents and issues, BIO urges PCORI to avoid soliciting comments on numerous issues simultaneously and suggests an additional round of review of the revised individual documents following the current comment period.

Additionally, PCORI has suggested that it plans to identify more specific “areas of priority funding,” as part of future iterations of the Research Agenda. To the extent that these areas are identified in a more specific manner than they have been for this first draft, BIO urges PCORI to solicit stakeholder feedback on this process through an open comment period or open public forum. For example, following finalization of the draft National Priorities and Research Agenda, PCORI may consider initiating a similar process to further refine and make more specific the first priority, “assessment of prevention, diagnosis, and treatment options.” BIO members would be interested in participating in sector-specific public forums where stakeholders would have the chance to share their thoughts on the clinical areas of interest to PCORI and the patients we serve, to identify PCOR questions and help to determine the appropriate methodologies required to answer those questions.

b. *PCORI’s comment process lacks transparency.*

While BIO is pleased to have the opportunity to provide comments on the PCORI National Priorities and Research Agenda, we are concerned that the comment process is not sufficiently transparent for stakeholders. Consequently, BIO urges PCORI to develop and describe in detail a systematic process regarding how PCORI will receive comments, and then synthesize and incorporate comments into the final drafts of the two documents currently under review. BIO suggests that PCORI prepare a document, such as those released by the Centers for Medicare and Medicaid Services (CMS) during comment periods, that compiles stakeholders’ comments and discusses the rationale behind PCORI’s final decisions regarding those comments.

Additionally, BIO urges PCORI to provide additional concrete details about the process of incorporating stakeholder comments into the final documents. PCORI continues to emphasize that the production of the final National Priorities and Research Agenda is an iterative process; however, the timeline for further revision periods is not publicly available. As stated above, BIO urges PCORI to institute a second round of stakeholder input, following the first revisions, before finalizing these documents. At least one additional round of public feedback will allow stakeholders to provide thoughtful comments, making the final documents – which serve as a basis for all PCORI work – as sound and complete as possible.

III. Conclusion

In conclusion, BIO recommends that PCORI take the following steps to improve the Draft National Agenda and Research Priorities:

- a. Provide greater specificity in the National Priorities and Research Agenda to ensure that the PCORI succeeds in its goal of building a solid and cohesive evidence base around specific research questions;
- b. Consider allocating more funding to research on improvements to health systems and delivery and the dissemination of research findings;

March 15, 2012

Page 5 of 5

- c. Balance outcomes improvement with medical innovation;
- d. Release documents sequentially to allow for the most considered feedback from stakeholders; and
- e. Increase the transparency of the comment process, with clear timelines and reasoned responses to stakeholder comments.

BIO appreciates the opportunity to offer these comments. We look forward to continuing to work with PCORI to address these critical issues in the future. Please feel free to contact me at 202-962-9200 if you have any questions or if we can be of further assistance. Thank you for your attention to this very important matter.

Respectfully submitted,

/s/

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