

September 2, 2011

Patient-Centered Outcomes Research Institute
Attn: PCOR Input
5185 MacArthur Blvd., NW
Suite 632
Washington, DC 20016

RE: Comments on Proposed Definition of PCORI

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

Yes

No

X Don't Know/ Not Sure

Additional Comments:

BIO believes that PCORI's proposed definition recognizes that different patients have different treatment needs and preferences, and research should therefore focus on informing patients on the relative merits of various clinical interventions. Patients may respond differently to the same intervention in ways that cannot be anticipated, and the needs of the individual must be taken into consideration. In this way, the proposed definition captures this variation, which is critical to achieving the patient-centeredness of PCORI's mission.

BIO is concerned, however, that the proposed definition fails to incorporate the personal exchange between a patient and his/her caregiver as to how this information may affect treatment decisions. When appropriately applied, comparative clinical effectiveness information is a valuable tool that, together with a variety of other types of medical evidence, can contribute to improving health outcomes and health care delivery. The research should strengthen, not weaken, the patient-caregiver relationship. BIO therefore urges PCORI to integrate into the definition the essential role of the caregiver.

While the definition captures the variation in patient needs, BIO encourages PCORI to more clearly integrate the critical role of the patient within its definition. The definition lacks the appropriate emphasis on early and direct patient engagement in this research process. The first sentence of the definition states that PCOR helps "people" make informed healthcare decisions. However, as this comment stresses, BIO believes comparative clinical effectiveness research information is best used to inform clinical decision making between a patient and his/her caregiver. Therefore, BIO urges PCORI to replace the word "people" with "patients and their caregivers" to specify for whom this information is targeted, and to be consistent with PCORI's mission.

Lastly, BIO urges PCORI to replace the word “harm” with “risk” wherever it may occur within the proposed definition. The use of “harm” indicates a particular, inevitable negative outcome. Conversely, “risk” implies only the potential of a negative outcome.



2. Is the definition consistent with the intent of the statute that established PCORI? A link to the statute is provided above.

Yes

X No

Don't Know/ Not Sure

Additional Comments:

The statute establishing PCORI consistently focuses on clinical comparative effectiveness research as a means to achieve improved health outcomes. It is important that PCORI preserve this focus as it establishes its research priorities and carries out its mission. In order to best meet the objectives of clinical comparative effectiveness research, PCORI must be cautious in how it explores the nonclinical determinants that affect health and likewise ensure that such examination is aligned with the intent and language of the statute.

Specifically, we are concerned with PCORI's consideration of "burden to individuals, resource availability, and other stakeholder perspectives."¹ As clarified in the rationale document, "some, but not necessarily all PCOR, should also focus on ways to optimize the outcomes of individuals while addressing issues that sometimes get in the way of achieving those outcomes."² Resource availability, also clarified in the rationale document, "includes infrastructure, system capacity, availability of human resources, and benefit coverage considerations for interventions based on an individual's insurer or other source of healthcare payment."³

BIO appreciates PCORI's recognition of the variety of factors that can influence a patient's decision to participate in the healthcare system, including but not limited to "co-payments, travel distance, treatment burden, and effect on family."⁴ These factors undoubtedly can affect the financial security, quality of life, and future well-being of the entire family. However, these are examples of considerations that must be evaluated by the patient and the caregiver and are outside the scope of PCORI's mission. These considerations may dilute the focus on patient outcomes. Clinical decision-making should not be informed by resource allocation policies, and the proposed definition suggests that these factors may be used as inputs in informing comparative clinical effectiveness research. We believe that this part of the proposed definition is inconsistent with the intent and language of the statute. The Affordable Care Act emphasizes comparative clinical effectiveness and explicitly rejects PCORI's use of cost-effectiveness.⁵ PCORI's commitment to patient-centeredness can easily become misguided if comparative clinical effectiveness information is used inappropriately, for example, as a means strictly to contain costs, rather than deliver health care value by improving patient health outcomes. The statute places restrictions on how Medicare can use comparative clinical effectiveness research in coverage decisions and specifically prohibits the program from relying solely on "evidence or findings from comparative clinical effectiveness research...in determining coverage, reimbursement, or incentive programs."⁶ Therefore, BIO strongly urges PCORI to re-word this portion of the definition to state "investigates (or may investigate) optimizing patient outcomes."

¹ Patient Centered Outcomes Research Institute. "Working Definition." Available at: <http://pcori.org/pcorinput.html>

² Patient Centered Outcomes Research Institute. "Rationale: Working Definition of Patient-Centered Outcomes Research." Available at: http://pcori.org/images/PCOR_Rationale.pdf

³ Id.

⁴ Id.

⁵ ACA, Pub. L. 111-148, Section 1182

⁶ ACA, Pub. L. 111-148, Section 1182(c)(1)

3. Is the definition broad enough to include the range of research that PCORI should fund?

Yes

No

Don't Know/ Not Sure

Additional Comments

BIO believes that PCORI's proposed definition encompasses the need to focus research on a wide variety of medical treatment options including preventive, diagnostic, therapeutic, and health delivery system interventions. We are pleased that the definition highlights PCORI's intention to conduct research on a broad array of interventions, extending well beyond pharmaceuticals, devices, and surgical procedures, to include other interventions including prevention, complementary and alternative medicine, and watchful waiting.

As PCORI's methodology committee, together with the board, evaluates and develops a framework of rigorous analysis, BIO urges both bodies to ensure that this research and other activities capture the critical balance that is required in improving patient health outcomes while also encouraging 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 improvement in patient health outcomes.

4. Does the definition adequately convey the rationale outlined in the rationale document? A link to the rationale document is provided above.

Yes

No

Don't Know/ Not Sure

Additional Comments:

BIO is pleased to see the inclusion of a rationale document, as it allows for stakeholder insight into PCORI deliberations and decisions, and provides additional background to inform this and future comments. Transparency and meaningful stakeholder involvement are critical to maintaining PCORI's credibility and as highlighted in our comments, we encourage PCORI to continue to foster stakeholder engagement in its activities at every step of the process.

While we recognize and appreciate PCORI's time and commitment in developing the proposed definition, we are concerned that the drafting process lacked transparency and limited the opportunity for public input. We also are concerned that the opportunity for input was limited to a subgroup of the Methodology Committee. In order to successfully fulfill its patient-centeredness mission, we encourage PCORI to consider integrating into its deliberation processes perspectives from patients and caregivers. This will ensure stakeholders, as well as the general public, that the patient perspective is being incorporated into PCORI's activities.

As conveyed in question 2, BIO urges PCORI to re-evaluate its consideration of "burden to individuals, resource availability, and other stakeholder perspectives"⁷ in its proposed definition. While the rationale document provides valuable insight into the underlying intent of this part of the definition, we are concerned that this language could be misinterpreted in a way that is inconsistent with PCORI's purpose.

⁷ Patient Centered Outcomes Research Institute. "Working Definition." Available at: <http://pcori.org/pcorinput.html>

For example, while we acknowledge that an individual's economic circumstances may impact a patient's decision to undergo a particular medical intervention, it should have no place in the comparative clinical research that PCORI is mandated to execute. We are concerned that this language could be easily misinterpreted to endorse cost-based research, which would compromise patient care. We strongly urge PCORI to keep the framework on which it bases comparative clinical effectiveness research separate from the resources involved in financing patient care. As stated in Question 2, BIO encourages PCORI to re-word this portion of the definition to state "investigates (or may investigate) optimizing patient outcomes."

5. Please use the following space to provide any additional comments you have about the definition.

Additional Comments

BIO appreciates the opportunity to comment on PCORI's proposed definition of PCOR. BIO strongly supported the creation of PCORI to conduct comparative clinical effectiveness research and maintains an ongoing desire to see the Institute successfully established. In order for PCORI to succeed, the Institute must maintain independent credibility which is critical to developing 'buy in' with patients and caregivers. BIO applauds the Institute for making clear in its definition that clinical comparative effectiveness information should inform clinical judgment and individual needs in medical decision-making, recognizing that patients may respond differently to the same intervention in ways that cannot be anticipated, and therefore must not be used as a "one size fits all" approach.

As it continues to develop its research priorities, BIO encourages PCORI to establish a process that allows for meaningful public input and participation, similar to the federal rulemaking process. This may entail: a preamble that describes the process and framework for the proposal; a proposal that includes questions highlighting specific areas needed for comment; and a final draft that includes generalized responses to related comments which informs the stakeholder community of PCORI's point of view and rationale for choosing, or not, various options. Transparency and meaningful stakeholder involvement are critical to maintaining PCORI's credibility.