

## **Patient-Centered Outcomes Research Definition Revision: Response to Public Input**

**CONSENSUS DEFINITION as of February 15, 2012**

### **Patient-Centered Outcomes Research Working Definition**

Patient-Centered Outcomes Research (PCOR) helps people and their caregivers communicate and make informed health care decisions, allowing their voices to be heard in assessing the value of health care options. This research answers patient-centered questions such as:

1. “Given my personal characteristics, conditions and preferences, what should I expect will happen to me?”
2. “What are my options and what are the potential benefits and harms of those options?”
3. “What can I do to improve the outcomes that are most important to me?”
4. “How can clinicians and the care delivery systems they work in help me make the best decisions about my health and healthcare?”

To answer these questions, PCOR:

- Assesses the benefits and harms of preventive, diagnostic, therapeutic, palliative, or health delivery system interventions to inform decision making, highlighting comparisons and outcomes that matter to people;
- Is inclusive of an individual’s preferences, autonomy and needs, focusing on outcomes that people notice and care about such as survival, function, symptoms, and health-related quality of life;
- Incorporates a wide variety of settings and diversity of participants to address individual differences and barriers to implementation and dissemination; and
- Investigates (or may investigate) optimizing outcomes while addressing burden to individuals, resource availability, and other stakeholder perspectives.

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## Public Comments and Proposed Response:

***Comment:*** PCOR is defined too broadly and tries to take on too much with respect to subpopulations

***Response:*** No change is recommended.

Several commentators expressed concern that the draft definition of PCOR was either too broad or too narrow, in particular, too specific about PCOR for individuals or populations of individuals. Other comments recognized the importance of subpopulations and populations for which genomics and personalized therapies can inform the impact of health care interventions on patient outcomes. PCORI recognizes that the state-of-the-art and science of personalized medicine, research feasibility, and applicability of research findings to different populations are important issues to address in the definition of PCOR. One goal of the definition of PCOR is to recognize that research that addresses subpopulations is important and that the definition should encourage research that addresses the needs of these groups and allows for research on personalized interventions as they become available.

***Comment:*** PCOR needs broader focus: psychosocial, socio-ecological; community-based and non-clinician supported services; public health; supportive (end of life) care

***Response:*** A change is recommended. “Palliative” care was added to the definition.

Several respondents commented that they thought the draft definition was too narrow and restrictive with regard to intervention, clinical vs. non-clinical, setting, cross-cutting issues, and type of research. PCORI is interested in a definition of PCOR that supports research of different types (experimental, non-experimental, systematic review, etc.), that investigates different settings of care, and diversity of interventions and the study designs to adequately study them. The draft definition does not preclude non-clinical research and addresses the importance of communities and non-traditional health care settings. Because of the unique role palliative or supportive care plays, it is added to the definition specifically: “Assesses the benefits and harms of preventive, diagnostic, therapeutic, palliative, or health delivery system interventions to inform decision making, highlighting comparisons and outcomes that matter to people.”

**Comment:** The definition should address health literacy.

**Response:** A change is recommended. The preamble now reads, “Patient Centered Outcomes Research (PCOR) helps people and their caregivers communicate and make informed health care decisions...” Question 4 now reads, “How can clinicians and the care delivery systems they work in help me make the best decisions about my health and healthcare?”

There are many characteristics of individuals that need to be considered...” to help people and their caregivers make informed health care decisions and allow their voice to be heard in assessing the value of health care options.” Health literacy is certainly one of the important characteristics that determine informed decision making and is a topic that will be addressed in PCORI-supported research. The draft definition has been modified in these two places to acknowledge the role of the care delivery systems in helping optimize decision making (and this might include addressing issues of literacy) and the preamble now focuses on PCOR’s role in improving communication (a component of which may include literacy and numeracy).

**Comment:** The definition should clarify that PCOR and CER are compatible; the definition should be about CER.

**Response:** No change is recommended.

The definition of PCOR includes many components of CER but is intended to be broader to also include other focuses and other research methodologies.

**Comment:** Comments related to cost, resources, value are not appropriate for the definition to include optimizing outcomes/addressing burdens/resources. Alternatively, comments also suggested that the definition should focus on costs but should not make reference to value.

**Response:** A change is recommended, adding the word “availability” after resources in the second part of the definition.

The proposed definition does not mention cost, but several comments assumed term “resources” was being used to reflect costs. Some respondents believe that the statute creating PCORI restricts the measurement or analysis of cost in PCORI-sponsored research. Section 1182 of the statute places restrictions on certain measurements or analytical approaches for the use of PCORI-sponsored research by the Secretary of HHS, but it does not

place any restrictions on collection or measurement of cost or resource utilization information in general.

The word *availability* will be added to the definition so that it will read: “may also focus on optimizing outcomes while addressing burden to individuals, resource *availability*, and other stakeholder perspectives.” “Resource availability” also refers to infrastructure, workforce and supply resources and understanding the availability of these resources on patients when considering different treatment strategies and making treatment decisions. For example, when comparing strategy A (a new technology in limited supply, requiring highly specialized clinicians to execute, and utilizing supplies that are in limited production) compared to strategy B (existing technology, not requiring specialists to execute and without limited supplies) for the treatment of a condition, PCOR should include studies that take into account the availability of the new technology, workforce capable of offering that technology and supply limitations. This resource availability may have direct bearing on how from a patient’s perspective the technology compares to strategy B.

**Comment:** The definition should include personal responsibility for one’s health.

**Response:** A change is recommended. Question 4 now states “How can clinicians and the care delivery systems they work in help me make the best decisions about my health and healthcare?”

Comments about personal responsibility touched on 2 issues: The nature of participation in health care decisions, and the relevance of lifestyle to health. Several public comments suggested that personal responsibility in care should be accounted for in the definition (e.g. “Patients have the responsibility to seek care and be as well-prepared as possible with information, questions, and articulate what they want from the encounter.”). The definition uses the term “informed health care decisions” and indicates that these decisions are made by patients (“people”), not by the physician. To emphasize this point, the revised definition now reads, “Patient Centered Outcomes Research (PCOR) helps people and their caregivers communicate and make informed health care decisions...” which highlights bidirectional communication and the role of the individual in decision making. It also says that PCOR “allows (*patients*) voices to be heard...” The change underscores the importance of patient participation in decision making and emphasizes the role of the caregivers and the health care delivery systems they work in optimizing that decision making.

We do not think changes in the definition are needed to address lifestyle choices. Two of the 4 questions in the definition are relevant here. The 2<sup>nd</sup> question (“What are my options...?”)

brings out that lifestyle change is often an *option*. Lifestyle change is often part of the answer to the 3<sup>rd</sup> question (“What can I do to improve the outcomes...?”)

**Comment:** The definition now focuses on “people.”

- It should focus more narrowly on patients
- It should focus more on society and less on the individual patient

**Response:** No change is recommended.

The preamble to the definition says “Patient Centered Outcomes Research (PCOR) helps people and their caregivers make informed health care decisions and allows their voices to be heard in assessing health care options.” It will be determined over time the degree to which PCORI’s research agenda will focus on societal issues that come to impact individual patient decision making or patient issues that impact decision making. Public comments in this area showed a mix of opinions about which should be the greater focus of PCORI. This phrasing clears a path for PCORI’s research agenda to potentially include a focus on both.

In the preamble the definition speaks about “people” rather than patients. Including this phrase was intended to signal that health care decisions are being made all the time by people who variably do and do not consider themselves patients. People without known disease who are considering a screening test such as a mammogram or prostate exam need the best information they can get to guide their decisions about which strategy will help them accomplish the outcomes they value. A narrow focus on those who refer to themselves as patients might also miss out on opportunities for PCOR generating information about health care decision making related to preventing the diseases that cause us to be patients.

**Comment:** The definition should emphasize decision-making:

- It should emphasize the collaboration between patient and provider, mutual and shared decision-making
- It should emphasize research on patient decision-making

**Response:** A change is recommended. The preamble now reads “Patient Centered Outcomes Research (PCOR) helps people and their caregivers communicate ...” and Example question 4 has been modified to “How can clinicians and the care delivery systems they work in help me make the best decisions about my health and healthcare?”



This phrasing demonstrates the centrality of improved decision making in the institute. Including this language clears a path for PCORI’s research agenda to potentially include research in the most effective ways for patients and providers to communicate and collaborate, including evaluations of the implementation and effect of mutual and shared decision-making interventions.

***Comment:*** The definition should be expanded to include caregiver concerns and burdens.

***Response:*** A change is recommended. The preamble now states “Patient Centered Outcomes Research (PCOR) helps people and their caregivers communicate...”

Caregiver burden is one of many outcomes of great concern to PCORI for some conditions. We think that the specific endpoint “caregiver burden” is already include in “outcomes that matter to patients.” we also emphasize the role or caregivers in communicating and making decisions with clinicians.

***Comment:*** The definition needs a better balance and understanding of benefits and harms: “risk” should replace “harm.”

***Response:*** A change is recommended, and the word “potential” was added to the 2nd question.

There was a mix of negative and positive public feedback about the term “harm,” some favoring “risk” and other “disadvantages.” We feel the word “harm” is more descriptive of the specific risks involved in medical care and is a clearer alternative to the term “benefits.” We also added the term “potential” to acknowledge that these good or bad results are only possible outcomes in response to public feedback.

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