

NPC Comments on the Patient-Centered Outcomes Research Definition September 2, 2011

The National Pharmaceutical Council appreciates the Patient-Centered Outcomes Research Institute's (PCORI) efforts to solicit broad public input on the working definition of "patient-centered outcomes research" (PCOR). The National Pharmaceutical Council is a health policy research organization dedicated to the advancement of good evidence and science, and to fostering an environment in the United States that supports medical innovation. Founded in 1953 and supported by the nation's major research-based pharmaceutical companies, NPC focuses on research development, information dissemination, and education on the critical issues of evidence, innovation and the value of medicines for patients. NPC welcomes the opportunity to submit comments on the definition of PCOR, which is an important concept that will guide the Institute's future activities.

NPC's responses to the questions posed by PCORI are included below.

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

Don't Know/Not Sure. NPC welcomes the importance placed on "patient-centeredness." In testimony in 2009 before the Institute of Medicine and Federal Coordinating Council on CER, NPC highlighted the need for PCOR to provide evidence that encourages good decision making by health care professionals and patients, and recognizes and supports the provider and patient at the center of the decision making process.

Although it is a worthy goal to provide patient-specific information for all patients and health decisions, the research questions are infinite and the logistical aspects are not practical. Given research time and cost limitations, balancing the information needs of individual patients with information to enlighten population health management strategies that can inform and improve our nation's health care will be needed. These aspects may be clarified through the following recommendations:

1. We suggest clarifying the term "people" as patients, healthy individuals, and caregivers. We welcome the broad definition of people which includes both patients and individuals considering wellness and prevention options, however, the broad definition of "people" may be interpreted to include a variety of stakeholders including patients, caregivers, but also organizations involved in the delivery of health care.
2. Remove the words "the value of" in "assessing the value of health care options." How health care options may be valued will differ by stakeholder and may refer to individual preferences or even financial considerations.

2. Is the definition consistent with the intent of the statute that established PCORI?

Don't Know/Not Sure. NPC welcomes a PCOR definition which embraces the following elements of our testimony in 2009 and is consistent with the statute:

- Utilizes a full range of types and sources of evidence that consider both direct and indirect benefits to society, such as quality of life, patient functionality and economic productivity.
- Considers the needs of patients who may respond differently to medicines and treatments based upon age, genetic variation, co-morbidities, or patient preferences.

There are opportunities to further ensure the PCOR definition is consistent with the intent of the statute by providing additional clarity on how PCOR will “investigate (or may investigate) optimizing outcomes while addressing burden to individuals, resources, and other stakeholder perspectives.” This statement allows for varying interpretation. For example, burden may relate to adherence or compliance burden for an individual, staff or testing burden for a health care system, or economic or financial burden to a health system or payer which extend beyond the statute’s focus on clinical comparative effectiveness. Further clarification of which other stakeholder perspectives are appropriate is needed. NPC cautions against the consideration of economic value and cost as these may best be considered on a regional or local level where health care decision makers can more accurately incorporate variations in health technology acquisition costs.

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

No. NPC recommends broadening the range of research to include "productivity at work and home" as outcomes about which people notice and care.

According to a recent survey, health care decision-makers within large, self-insured employers are aware of comparative effectiveness research and its potential to answer critical questions about health care. Employers are very interested in health and productivity information and its inclusion would encourage their adoption and use of comparative effectiveness research. Additional data would close a gap that currently exists among employers: fewer than one-third of employers (or their vendors) currently use information about absence, disability and return to work in assessing the impact of various treatments, yet 70 percent indicate that such information would be “Important” or “Very Important.” At 76 percent, more employers chose PCORI than any other potential resource as the most reliable and trusted source for CER information.

The survey was published in the July 2011 edition of *Journal of Occupational and Environmental Medicine* (p. 708-710) and includes feedback from 75 employee benefit directors, medical directors and other health management professionals of large (5,000 or more employees), self-insured companies.

Finally, we recommend replacing the phrase the word “harms” with “risks.” Risk encompasses the likelihood of harm which may or may not be similar for all patients.

4. Does the definition adequately convey the rationale outlined in the rationale document?

Yes.

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

NPC and its member companies recommend that the working definition of patient-centered outcomes research evolve into a traditional definition format to increase the longevity and impact.

Traditionally, a formal definition includes 1) the term, 2) the group of objects of concept that are related, and 3) the special characteristics that make the term different from others in its group. While the unique characteristics of PCOR are explicit, the definition could be improved upon by emphasizing what it "is."

In summary, we suggest the definition be altered in the following manner:

- "Patient-Centered Outcomes Research is research that helps people, (both consumers, patients and their caregivers), make informed health care decisions and allows their voice to be heard in assessing health care options"
- Considers "risks" rather than "harms"
- Outcomes of "productivity at work and at home" are considered.

Thank you again for the opportunity to be a part of this critical dialogue. We stand ready to assist PCORI as it moves forward with its important activities.