



Patient-Centered Outcomes Research Institute

**Report from the Methodology Committee
To the Board of Governors**

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PCORI Methodology Committee

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Agenda

- Welcome & Introductions
- Administrative Matters (and Charter)
- What **Patient-Centered** Means in the Context of Our Work
- Preliminary list of **Critical Gaps** in Patient Centered Outcomes Research & **Criteria** used to for identifying these
- Next Steps



Administrative Issues

- » Minutes
- » Confidentiality Policy
- » Guidance on Media and Speaking Requests
- » Conflict of Interest
- » Sharepoint
- » Face-to-face Meeting Times

Ground Rules

- » Availability of Chair, Vice-Chair and Staff
- » Open honest debate
- » Share all contributed materials and comments
- » Fast pace
- » Inclusive, team – akin to Board
- » Seek consensus, may not always achieve
- » Alignment with Board direction and close integration with three subcommittees, especially Program Development Committee

Committee Charter

Purpose Statement- endorsed!!

Membership

Committee Operations

Responsibilities

Methodology Committee: Purpose

The Methodology Committee (“Committee”) shall make recommendations to the Board of Governors (“Board”) regarding **methods** for patient-centered outcomes research. This includes **guidance** about the **appropriate use** of methods in such research, including **methodological standards**, as well as establishing **priorities to address gaps** in research methods or their **application**.



What does Patient-Centered Outcomes Research mean in the context of our work?

Results from 4 break-out groups
Independent process informed by prior work
Work in progress
Drafts submitted for consideration/collaboration



Draft Definition 1:

*Patient-centered outcomes research seeks to **understand** and **improve** the effects of **healthcare** and **prevention** services on outcomes important to **all persons** with disease or **at risk** for disease considering **individual perspectives, needs, preferences, biological, environmental, behavioral, and cultural determinants of health.***



Draft Definition 2:

*Patient-centered outcomes research is targeted specifically to help patients live longer, live better, or preferably, both. It highlights and enhances the **fundamental right of patients to choose** specific interventions and strategies to **prevent, treat, or manage** conditions using **evidence-based** information on the relative **comparative** benefits and harms of different options. It also incorporates research on interventions that are targeted to **individual-level biological, environmental, behavioral, and cultural characteristics and preferences.***



Draft Definition 3:

*Patient-centered outcomes research is research that determines the **real world effectiveness** (results) of interventions on outcomes that **persons' care about**, including survival, function, symptoms, and health-related quality of life. PCOR includes **variability in individuals' risk** of experiencing the outcomes and likelihood of **benefit or harm** of the interventions.*



Draft Definition 4:

*Patient-centered outcomes research seeks to understand and improve the effects of health services. It incorporates **patient's perspectives**. By including **biological, environmental, behavioral, and cultural components** and in setting research priorities.*

*It must **optimize outcomes while taking account of multiple competing demands** of:*

- *Population health considerations*
- *Other stakeholder perspectives (payers, policymakers and industry)*
- *Barriers to implementation*

*Generates **guidance to enable patient participation and overcome barriers to implementation***

PCOR Methodology Gaps

Some Ideas which Emerged:

- **Implementation** – “Gulf between what we know and what we do”
- **Observational** studies vs. randomized control trials (RCT’s): when do we need to randomize? How to reduce bias in observational research?
- Developing appropriate tools for measuring **patient-reported outcomes**, i.e. which patient reported outcomes are appropriate in which contexts (e.g. symptoms, adverse reactions, global measures of health status, patient impressions of value, treatment compliance etc.
- Standardized item sets of **patient-reported outcomes** should be developed for specific contexts to enable subsequent cross-study analyses

PCOR Methodology Gaps

- Addressing **Bias**. Eg. Selective reporting, selective analyses in systematic reviews
- Benchmarking outcomes measurements across all diseases
- Effecting **behavior change in health care systems**
- Methods for **predicting and personalizing medicine**, including validation
- Appropriate methods for synthesizing evidence for **complex social interactions**
- How should we reflect **variability across individuals** in outcomes research
- **Health Equity**: best methods to ensure inclusiveness eg. in RCTs
- Methods to **disseminate** individualized evidence-based knowledge to enable better decisions and optimum self management



NEXT STEPS

Working Groups:

- Complete work on PCOR definitions in collaboration with PDC
- Finalize review of charter and submit to Board
- Work plan: objectives, tactics, deliverables, dates, accountable
- Prioritize methodology gaps (define criteria)
- Propose ideas for white papers
- Review and revise ground rules



THANK YOU !