



PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

# PCORI Methodology Standards: Academic Curriculum



JOHNS HOPKINS  
SCHOOL *of* MEDICINE

## Introductory Lecture



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# Outline

1. Introduction to PCORI and the PCORI Methodology Committee
2. Definitions and Terminology
3. The PCORI Methodology Report and Standards
4. Aims of the PCORI Methodology Standards Academic Curriculum

# Introduction to PCORI and the PCORI Methodology Committee

# About PCORI

- PCORI is an independent, nonprofit organization authorized by The Patient Protection and Affordable Care Act (PPACA) in 2010
- PCORI is charged with funding research aimed at giving patients and their caregivers a better understanding of prevention, treatment, and care options and informing the science that supports those options
- PCORI is dedicated to funding research that will improve the quality and relevance of evidence to help patients, caregivers, clinicians, insurers, and policy makers make informed health decisions
- PCORI is committed to transparency and rigorous processes
  - ▶ It uses a variety of forums that emphasize meaningful engagement from patients and a broad range of stakeholders throughout the entire research process to guide and enhance its work

# Five National Priority Areas Upon Which PCORI Is Focusing Efforts

1. Assessment of prevention, diagnosis, and treatment options
2. Improving healthcare systems
3. Communication and dissemination research
4. Addressing disparities
5. Accelerating patient-centered outcomes research (PCOR) and comparative-effectiveness research (CER) methods

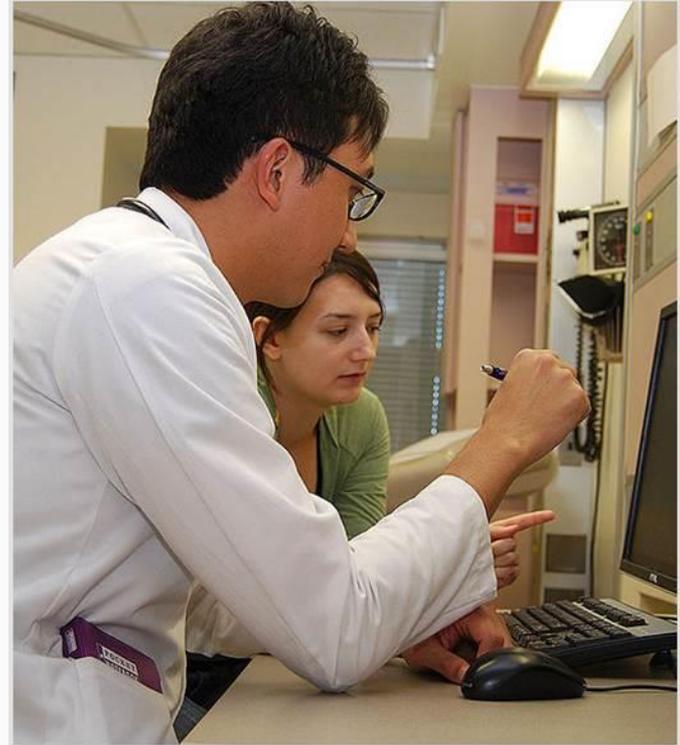
# The PCORI Methodology Committee

- The PPACA required the establishment of a Methodology Committee tasked with making recommendations to the PCORI Board of Governors about methods for PCOR
- The PCORI Methodology Committee was formed in 2011 to “develop and improve the science and methods of comparative clinical effectiveness research”
- The Committee provides critical guidance to PCORI in advancing its mission, and to the research field more broadly

## Definitions and Terminology

# What Is Patient-Centered Outcomes Research (PCOR)?

- PCOR helps people and their caregivers communicate and make informed healthcare decisions, allowing their voices to be heard in assessing the value of healthcare options
- PCOR has the following characteristics:
  - ▶ Compares important clinical management options
  - ▶ Evaluates the outcomes that are most important to patients
  - ▶ Addresses implementation of the research findings in clinical care environments
  - ▶ Actively engages patients and key stakeholders throughout the research process



# What Is Comparative-Effectiveness Research (CER)?

- CER “compares the benefits and harms of alternative methods to prevent, diagnose, treat, and monitor a clinical condition or to improve the delivery of care” (Institute of Medicine, 2009)
- PCORI has developed a program of PCOR that meets this goal by putting a priority on research that compares different clinical options and then measures their clinical outcomes—using outcomes that are meaningful to patients
- PCORI was established to help people make informed healthcare decisions and improve healthcare delivery and outcomes by producing CER that is guided by patients, caregivers, and the broader healthcare community
- This research, which takes into account patients’ values and preferences, helps individuals and their caregivers make informed healthcare decisions

# What Is a Methodology Standard?

“A process, action, or procedure for performing PCOR that is deemed essential to producing scientifically valid, transparent, and reproducible results. A standard may be supported by scientific evidence. When such evidence is unavailable, a standard may be endorsed by reasonable expectation that the standard helps achieve the desired level of quality in PCOR or by broad acceptance of the practice in PCOR ....The research practices recommended by the standard can be feasibly implemented.”

—Institute of Medicine, 2011

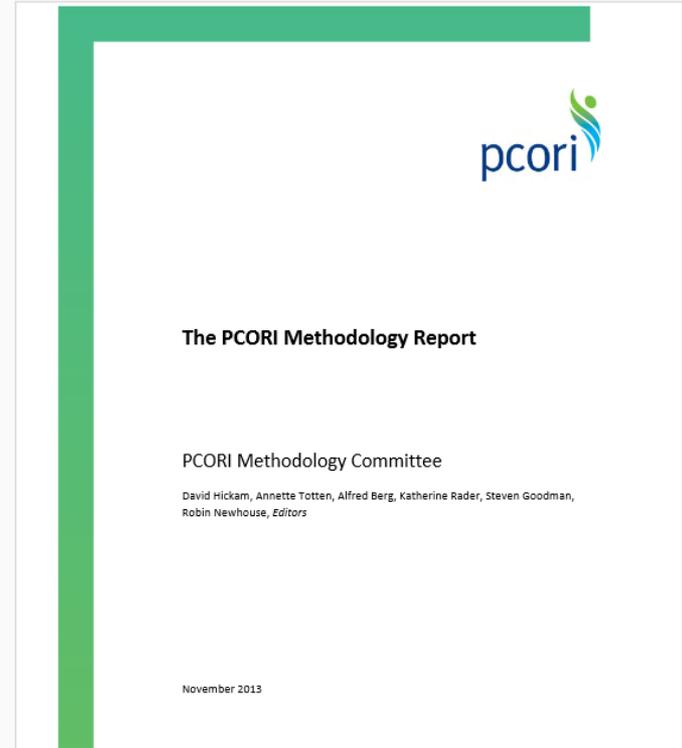
# The PCORI Methodology Report and Standards

# The PCORI Methodology Report

- In accordance with PCORI's authorizing legislation, a draft Methodology Report was submitted to the PCORI Board of Governors in May 2012
- A period of public comment followed, in which extensive feedback was received from the healthcare community (July through September 2012)
- A revised PCORI Methodology Report was adopted by the PCORI Board in November 2012 and then published in December 2012, with minor editorial revisions made the following year
- The final version of the PCORI Methodology Report was approved by the PCORI Board of Governors and published in November 2013

# The PCORI Methodology Report

- The PCORI Methodology Report provides recommendations and a framework for best research practices in PCOR and CER
- The report includes vignettes that illustrate different ways that good methodology makes a difference to patients and their care, including stories of patients' experiences navigating choices and weighing options
- Examples of published studies that capture the impact of good methodology are also included



# Why Are Methodology Standards Necessary for PCOR?

- Departures from basic good research practices are partially responsible for the mismatch between the quality and relevance of the information that research provides and the information patients need to make informed clinical decisions
- There are substantial inconsistencies in how methods are applied in practice (Helfand, et al., 2011; Lohr, 2007; Schneeweiss, et al., 2012)
- And it's the law:
  - ▶ Patient Protection and Affordable Care Act, Subtitle D, Paragraph (6)(C)
    - “Methodological standards shall provide specific criteria for internal validity, generalizability, feasibility and timeliness of research and for health outcomes measures, risk adjustment, and other relevant aspects of research and assessment with respect to the design of research.”

## Sources:

Helfand, M., et al. (2011). *Clinical and Translational Science*, 4(3), 188-198. <http://doi.org/10.1111/j.1752-8062.2011.00282.x>

Lohr, K. N. (2007). *Medical Care*, 45(10 Supl 2), S5-8. <http://doi.org/10.1097/MLR.0b013e31812714b6>

Schneeweiss, S., et al. (2012). *Pharmacoepidemiology and Drug Safety*, 21 Suppl 2, 1-5. <http://doi.org/10.1002/pds.3270>

# The Promotion of Transparency in PCOR

- Many of the standards promote transparency by requiring such best practices as:
  - ▶ Preparing detailed research protocols (including a well-formulated research question)
  - ▶ Adhering to guidelines for registering studies and reporting results
  - ▶ Assessing appropriate methods for data sharing and giving proper credit to those sharing protocols, code, and data

# Promoting Uptake of the PCORI Methodology Standards

- PCORI is pursuing a comprehensive, coordinated approach to promoting the uptake of the Methodology Standards, including:
  - ▶ Engaging all stakeholders who might use the standards
  - ▶ Collaborating with other organizations and initiatives to strengthen research practices and facilitate use of the standards
  - ▶ Creating reporting and surveillance mechanisms
  - ▶ *And ...* disseminating this curriculum

## Aims of the PCORI Methodology Standards Academic Curriculum

# To Whom Is the Academic Curriculum Directed?

- Faculty and staff investigators at universities who conduct PCOR/CER
- Investigators at foundations or patient-advocacy organizations who fund or conduct PCOR/CER
- Master's level and doctoral students who are planning to conduct PCOR/CER
- Staff of regulatory bodies, such as the US Food and Drug Administration (FDA), which is incorporating more PCOR into its evaluation of drugs and devices through their life cycles
- Project managers, nursing staff, or research staff conducting PCOR

# Goals of the PCORI Methodology Standards Academic Curriculum

- To help investigators gain the knowledge, skills, and abilities to apply the Methodology Standards to research in progress
- To provide guidance regarding attending to the standards when designing new studies
- To provide learners with general knowledge on the topics included in the Methodology Standards

# Organization

- This curriculum is divided into Categories that match the Methodology Standards
- Each Category has a set of lecture modules, each of which runs from 5 to 20 minutes
- We encourage watching and listening to the modules within each category sequentially, but this is not entirely necessary
- Each Category ends with a series of questions that the learner can use as a self-assessment
- You will find that some Categories require more baseline epidemiological and statistical knowledge than others

# Acknowledgments

- We gratefully acknowledge use of the following document:
  - ▶ Selby, J. V., Hickam, D., Mittman, B., Tunis, S. (June 4, 2013). *Using the PCORI Methodology Standards to Generate Robust, Relevant, and Timely Evidence for Patient-Centered Outcomes Research*. Academy Health Presentation.

# References

- Helfand, M., Tunis, S., Whitlock, E. P., Pauker, S. G., Basu, A., Chilingerian, J., et al., and the Methods Work Group of the National CTSA Strategic Goal Committee on Comparative Effectiveness Research. (2011). A CTSA agenda to advance methods for comparative effectiveness research. *Clinical and Translational Science*, 4(3), 188-198. <http://doi.org/10.1111/j.1752-8062.2011.00282.x>
- Institute of Medicine (US). (2009). *Initial National Priorities for Comparative Effectiveness Research*. Washington, DC: National Academies Press.
- Institute of Medicine (US). (2011). *Finding What Works in Health Care: Standards for Systematic Reviews*. (J. Eden, L. Levit, A. Berg, & S. Morton, Eds.). Washington, DC: National Academies Press.
- Lohr, K. N. (2007). Emerging methods in comparative effectiveness and safety: symposium overview and summary. *Medical Care*, 45(10 Supl 2), S5-8. <http://doi.org/10.1097/MLR.0b013e31812714b6>
- Schneeweiss, S., Seeger, J. D., & Smith, S. R. (2012). Methods for developing and analyzing clinically rich data for patient-centered outcomes research: an overview. *Pharmacoepidemiology and Drug Safety*, 21 Suppl 2, 1-5. <http://doi.org/10.1002/pds.3270>