Patient-Centered Outcomes Research Institute

Grayson Norquist, PCORI Board of Governors
Sherine Gabriel, PCORI Methodology Committee (chair)

Institute of Medicine
Clinical Effectiveness Research Innovation Collaborative of the Roundtable on Value & Science-Driven Health Care

July 25, 2011
Objectives

I. Overview of PCORI
II. Methodology Committee
III. PCORI and Health Disparities
PCORI’s Origin

Public Law 111–148  
111th Congress

An Act
Entitled The Patient Protection and Affordable Care Act.

Be it enacted by the Senate and House of Representatives of the United States of America in Congress assembled,

SECTION 1. SHORT TITLE; TABLE OF CONTENTS.
(a) SHORT TITLE.—This Act may be cited as the “Patient Protection and Affordable Care Act.

Subtitle D—Patient-Centered Outcomes Research

SEC. 6301. PATIENT-CENTERED OUTCOMES RESEARCH.
(a) IN GENERAL.—Title XI of the Social Security Act (42 U.S.C. 1301 et seq.) is amended by adding at the end the following new part:

“PART D—COMPARATIVE CLINICAL EFFECTIVENESS RESEARCH

“COMPARATIVE CLINICAL EFFECTIVENESS RESEARCH
PCORI’s Core Duties

- Review and assess existing research
- Identify national research priorities and agenda
- Carry out research
- Develop and update methodological standards
- Disseminate
PCORI Resources

FY 2010 - 2012
• $210 million from the Patient-Centered Outcomes Research Trust Fund

FY 2013
• $150 million from the Patient-Centered Outcomes Research Trust Fund
• $1 fee per Medicare and private health insurance beneficiary
• Estimated at $325 million

FY 2014 - 2019
• $150 million from the Patient-Centered Outcomes Research Trust Fund
• $2 fee per Medicare and private health insurance beneficiary
• Estimated at $500 million
PCORI Board of Governors

Debra Barksdale, PhD, RN  
Kerry Barnett, JD  
Lawrence Becker  
Carolyn Clancy, MD  
Francis Collins, MD, PhD  
Leah Hole-Curry, JD  
Allen Douma, MD  
Arnold Epstein, MD  
Christine Goertz, DC, PhD  
Gail Hunt  
Robert Jesse, MD, PhD  
Harlan Krumholz, MD  
Richard E. Kuntz, MD, MSc  
Sharon Levine, MD  
Freda Lewis-Hall, MD  
Steven Lipstein, MHA (Vice Chair)  
Grayson Norquist, MD, MSPH  
Ellen Sigal, PhD  
Eugene Washington, MD, MSc (Chair)  
Harlan Weisman, MD  
Robert Zwolak, MD, PhD

- Health services researchers
- Federal and state public health officials
- Pharmaceutical, device, and diagnostic manufacturers
- Private payers
- Physicians, nurses and providers
- Caregivers
- Patients and health care consumers
Goals for First Two Years

• Engage patients, and stakeholders in a two-way dialogue

• Assess current state of comparative effectiveness and outcomes research

• Assemble permanent professional staff

• Fund Tier 1 pilot projects to inform PCORI priorities and identify knowledge gaps

• Complete Methodology Comm. report
Goals for First Two Years

• Disseminate findings to the public.
• Support expansion of research capacity.
• Improve type of evidence-based information available for decisions on prevention, diagnosis and treatment.
Patient and Stakeholder Engagement Strategies

We provide patients and stakeholders multiple opportunities to have meaningful input in PCORI’s work through direct outreach, online engagement, special events and meetings.

- Stakeholder Discussion Forums
- Patient Listening Sessions and Stakeholder Group Meetings
- Upcoming Board meeting sites:
  - Seattle, WA (Sept. 19-20)
  - New Orleans, LA (Nov. 14-15)
- Online through pcori.org.
Research: Landscape Reviews

• Review existing comparative effectiveness and outcomes research
• Assess stakeholder engagement methods and efforts by other organizations
• Analysis of current dissemination capacity
• Evaluation of existing methodologies for this research
• Review of existing research organizations and networks for potential collaboration
Landscape Reviews: Update

Progress:

- CER Inventory Project
- Global CER Organization Inventory
- Peer Review Model
  - Engaging Stakeholder in Peer-Review
- AHRQ Dissemination Efforts
- AHRQ CER Capacity Efforts
- Methodology Committee Landscape Reviews
Research: Tier 1 Pilot Projects

• Designed to inform the development of national research priorities and research agenda

• Projects focused on developing, testing, and/or evaluating methods or approaches that:
  – Inform the National Priorities
  – Bring together different stakeholders
  – Translate research into practice
  – Identify gaps in relation to issues for disadvantaged populations
  – Identify predictors of patient outcomes
PCORI Methodology Committee
Background & Members

Named by the Comptroller General of the Government Accountability Office - January 2011

- Clinical Researchers
- Epidemiologists
- Health Services Researchers
- Statisticians
- Public & Private Institutions
- AMC, VA, NIH & AHRQ
- Varied Clinical & Scientific Disciplines

Naomi Aronson, PhD
Ethan Basch, MD, MSc
Alfred Berg, MD, MPH
David Flum, MD, MPH
Sherine Gabriel, MD, MSc (Chair)
Steven Goodman, MD, PhD
Mark Helfand, MD, MS, MPH
John Ioannidis, MD, DSc
Michael Lauer, MD
David Meltzer, MD, PhD
Brian Mittman, PhD
Robin Newhouse, PhD, RN
Sharon-Lise Normand, MSc, PhD (Vice Chair)
Sebastian Schneeweiss, MD, ScD
Jean Slutsky, PA, MSPH
Mary Tinetti, MD
Clyde Yancy, MD, MSc
“(6) ESTABLISHING METHODOLOGY COMMITTEE.—
“(A) IN GENERAL.—The Institute shall establish a standing methodology committee to carry out the functions described in subparagraph (C).

“(C) FUNCTIONS.—Subject to subparagraph (D), the methodology committee shall work to develop and improve the science and methods of comparative clinical effectiveness research by, not later than 18 months after the establishment of the Institute, directly or through subcontract, developing and periodically updating the following:

“(i) Methodological standards for research
“(ii) A translation table
• The **Methodology Committee** shall make recommendations regarding **methods** for patient-centered outcomes research.

• This includes **guidance** about the **appropriate use of methods** in such research, methodological standards, as well as **establishing priorities to address gaps** in research methods or their application.
Methodology Committee Work Cycle

Review (Update) Landscape: Methodological Standards & Translation Table

Advance Methodological Research and Innovation to Address Knowledge Gaps

Identify Critical Gaps in Knowledge and/or Implementation
Operations

PCORI Board of Governors

Methodology Committee

Patient-Centeredness Work Group

Research Prioritization Work Group

Research Methods Work Group

Workflow

Board Committees

Landscape Reviews & Workshops on Methodological Standards & Translation

Critical Gaps in knowledge or implementation identified

Advance methodological research & innovation to address Gaps
Achieving PCORI Goals

Methodology Committee Working Groups

Patient-Centeredness
Ethan Basch (chair), Mary Tinetti (co-chair), Naomi Aronson & Brian Mittman

**Identify** methodological standards for incorporating the patient perspective into three key areas:

1. Development and prioritization of research questions
2. Design of study components, including selection of population, interventions, comparators, outcomes, and timing/setting
3. Processes of clinical decision-making/care delivery

The goal of this work is that PCOR will address issues relevant to patients, ultimately impacting meaningfully on their experiences with disease, treatment, and delivery systems.
Achieving PCORI Goals

Methodology Committee Working Groups

Research Prioritization
David Meltzer (chair), John Ioannidis, Jean Slutsky, & Clyde Yancy

Provide guidance concerning the use of methods to inform the establishment of research prioritization approaches that best fulfill PCORI’s mission, including:

1. Topic generation
2. Gap analysis in systematic reviews,
3. Value of information analysis
4. Peer review
Achieving PCORI Goals

Methodology Committee Working Groups

Research Methods
Steven Goodman (chair), Robin Newhouse (co-chair), Michael Lauer, Sebastian Schneeweiss, and Sharon-Lise Normand

**Identify** Standards for the use of data, design, and statistical analyses to conduct patient centered outcomes research through:

1. Review existing data systems and strategies designed to permit exploration of causal questions using real-time practice data
2. Review methodological standards issued by expert bodies
3. Create evidence and translation tools based on findings from (1) and (2)
Achieving PCORI Goals

Methodology Committee Working Groups

**Report Assimilation**
- Mark Helfand (chair), Alfred Berg, David Flum, Sharon-Lise Normand & Sherine Gabriel

**Assimilate** work products into a cohesive final report

Develop interactive tools for disseminating the results to multiple audiences
Patient Relevance
Drowning in a sea of information
The Critical Role of Methodological Standards

Providing methodological standards for PCOR and making these accessible to the public will allow people to understand the healthcare options they face and make better more personalized decisions.

Understanding the relevance of the methods

Comparing the options

Making better, more personalized decisions
ACCOMPLISHMENTS | Moving Forward

MC Face-to-Face Meetings at Board of Governors

- MC Face-to-Face Meetings
- MC Charter
- MC Work Plan
- PCOR Definition
- PCOR Methodological Standards & Translation Table

Commission Papers
- Sponsor Workshops
- Fund Tier 1 Grants

2011

FEB MAR APR MAY JUN JUL

MC Formed

Patient Centered Research Methods Research Prioritization

Integrating Methods Patient Centered EHR Data Problems

Research Gaps
Patient-Centered Outcomes Research Institute

PCORI seeks your input on the definition of “Patient-Centered Outcomes Research”
Working Definition of PCOR

Patient Centered Outcomes Research (PCOR) helps people make informed health care decisions and allows their voice to be heard in assessing the value of health care options. This research answers patient-focused questions:

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 benefits and harms of those options?”
3. “What can I do to improve the outcomes that are most important to me?”
4. “How can the health care system improve my chances of achieving the outcomes I prefer?”

To answer these questions, PCOR:

• Assesses the benefits and harms of preventive, diagnostic, therapeutic, 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, resources, and other stakeholder perspectives.
PCORI’s Role in Eliminating Health Disparities
“IDENTIFYING RESEARCH PRIORITIES.—The Institute shall identify national priorities for research, taking into account factors of disease incidence, prevalence, and burden in the United States (with emphasis on chronic conditions), gaps in evidence in terms of clinical outcomes, practice variations and health disparities in terms of delivery and outcomes of care...”

PCORI Law on Disparities

GAO’s Annual Review Includes Focus:

“Such review shall include an analysis of the extent to which research findings are used by health care decision-makers, the effect of the dissemination of such findings on reducing practice variation and disparities in health care, and the effect of the research conducted and disseminated on innovation and the health care economy of the United States.”

Strategies

- Broad and comprehensive input
- Methodological innovation
- Collaboration
- Develop priorities
- Focus on some key areas initially
- New paradigm
Connect with PCORI

Website: pcori.org

Email: info@pcori.org

- Provide feedback
- Submit inquiries
- Join PCORI’s mailing list

Live webcast provided for all Board meetings.