Update on the Patient-Centered Outcomes Research Institute

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The purpose of the Institute is to assist patients, clinicians, purchasers, and policymakers in making informed health decisions by advancing the quality and relevance of evidence concerning the manner in which diseases, disorders, and other health conditions can effectively and appropriately be prevented, diagnosed, treated, monitored, and managed through research and evidence synthesis that considers variations in patient subpopulations and the dissemination of research findings with respect to the relative health outcomes, clinical effectiveness, and appropriateness of medical treatments, services.
MISSION STATEMENT

(adopted July 17, 2011)

The Patient-Centered Outcomes Research Institute (PCORI) helps people make informed health care decisions – and improves health care delivery and outcomes – by producing and promoting high integrity, evidence-based information - that comes from research guided by patients, caregivers and the broader health care community.
Taking the Patient and Stakeholder Perspective Seriously

- Patients and other relevant stakeholders must be included as members of research teams and involved in all aspects of the research project - from planning to dissemination.

- Applications are evaluated and scored on patient-centeredness and on patient-stakeholder engagement as part of the review process.

- 30% of reviewers on each review committee are trained patient or stakeholder reviewers.
PCORI’s National Priorities and Research Agenda

Assessment of Prevention, Diagnosis, Treatment Options
- Comparisons of alternative clinical options; identifying patient differences in response to therapy; studies of patient preferences for various outcomes

Improving Healthcare Systems
- Improving system support of patient self-management, coordination of care for complex conditions, access to care; comparing alternative strategies for workforce deployment.

Communication & Dissemination Research
- Understanding and enhancing shared decision-making; alternative strategies for dissemination of evidence; exploring opportunities to improve patient health literacy

Addressing Disparities
- Understanding differences in effectiveness across groups; understanding differences in preferences across groups; reducing disparities through use of findings from PCOR

Accelerating PCOR and Methodological Research
- Improving study designs and analytic methods of PCOR; building and improving clinical data networks
#1 Investigator-Generated Pathway to Research

PCORI issues broad funding announcements.

Researchers partner with stakeholders to generate questions.

Researchers, stakeholders apply review criteria in their applications.

Peer review prioritizes applications by level of alignment with criteria.

Diverse Portfolio addressing high-priority questions.
First PCORI Funding Announcements (PFAs) – May 2012

PFAs

Communication and Dissemination Research

The Patient-Centered Outcomes Research Institute is accepting applications for research to better understand the causes of disease, improve care, and help people make informed health care decisions.

PFAs

Research Agenda

The first four National Priorities: Assessment of Prevention, Diagnosis, and Treatment Options; Improving Healthcare Systems; Communication and Dissemination Research; Addressing Disparities.
Requirements of Applications to Broad PFAs

- Address a question relevant to patients and consider patient-relevant outcomes
- Be comparative
- Assess for differences in treatment effectiveness across subgroups of study population
- Include relevant patients and other stakeholders on research team
- Discuss potential for dissemination and implementation
- NOT include cost-effectiveness analyses or treat costs of care as an outcome that is compared
| 1. | Impact of the Condition on the Health of Individuals and Populations |
| 2. | Innovation and Potential for Improvement |
| 3. | Impact on Health Care Performance |
| 4. | Patient-Centeredness |
| 5. | Rigorous Research Methods |
| 6. | Inclusiveness of Different Populations |
| 7. | Research Team and Environment |
| 8. | Efficient Use of Research Resources |
Cycle 1 Response to Broad PFAs
Proposals Advanced to Phase II:

Condition Studied

- Acute Care: 24
- Cancer: 42
- Cardiac Health: 31
- Chronic Conditions: 71
- Diabetes: 33
- Mental Health: 30
- Pain: 16
- Rare Disease: 8
- Vascular Health: 9
#2 Patient/Stakeholder-Led Pathway to Research

PCORI and stakeholders generate and prioritize questions based on review criteria.

PCORI issues specific, funding announcements for highest priority topics.

Researchers and stakeholders develop responsive proposals.

Peer review prioritizes applications by level of alignment with criteria.

Diverse portfolio addressing high-priority questions.
Developing PCORI’s Research Prioritization Process

**Topic Generation**

- Patients & Stakeholders:
  - Web Page
  - Social Media
  - Workshops
  - Guidelines and Systematic Reviews
  - Direct Communication with Stakeholder Groups
- PCORI
  - Continuous Portfolio Review
- Supported by AHRQ:
  - Systematic Review

**Gap Confirmation**

(In Collaboration With AHRQ)

**Research Prioritization**

(Multi-stakeholder Advisory Panels)

**Final Selection Specific PFAs**

(PCORI Board)
Multi-stakeholder Advisory Panels will assist PCORI staff and Board in:

- Modeling robust patient and stakeholder engagement efforts
- Identifying, refining and prioritizing specific research questions
- Provide other scientific or technical expertise – e.g., for clinical trials and for rare diseases
- Other questions that may arise relevant to PCORI’s mission and work – e.g., refinement of research agenda; dissemination of PCORI research findings
Targeted Funding Announcements

- May call for single or multiple projects
- May be organized as cooperative agreements or as “contracts”
- Will seek to identify opportunities to co-fund
- Will require that projects meet PCORI criteria for patient-centeredness and engagement
Building infrastructure for PCOR and a learning health care system
Desirable Characteristics of a Data Infrastructure for PCOR

- Ideal Data Infrastructure for PCOR
  - Covers large, diverse, defined populations from usual care settings
  - Allows for complete capture of longitudinal data
  - Active patient and clinician engagement in governance of data use
  - Is efficient in terms of costs for data acquisition, storage, analysis
  - Linkages to health systems for rapid dissemination of findings
  - Capable of randomization—at individual and cluster levels

Covers large, diverse, defined populations from usual care settings
Two Distinct (Complementary) Approaches to Building Research Infrastructure Emerge

PCORI National Workshop to Advance Use of Electronic Data
July 2-3, 2012
Funders, Models, and Opportunities

**ONC**
- Meaningful Use
- EHR Certification programs
- SHARP Program
- BEACON Communities

**FDA**
- Sentinel
- OMOP

**AHRQ**
- DRNs
- PBRNs
- Registries
- SPAN
- PROSPECT
- EDM Forum

**NIH**
- CTSA
- Collaboratory
- CRN, CVRN
- ClinicalTrials.gov
- eMERGE Network
- PROMIS/ NIH - Snomed-CT, LOINC

**VA**
- VistA
- iEHR (2017)

**IOM**
- 2011 Report: *Digital Infrastructure for the Learning Health System: The Foundation for Continuous Improvement in Health and Health Care*

**Payers**
- hmo research
- OPTUMInsight™
- WELLPOINT

**Specialty Societies**
- ACC (American College of Cardiology)

**Industry**
- Medtronic
- Mainspring Data Express

**Innovators and Entrepreneurs**
- patientslikeme
Join Us at PCORI.org