Research Done Differently: PCORI’s Approach to Patient-Centered Outcomes Research

Joe Selby, MD, MPH
Executive Director, PCORI

Today’s Agenda

- About PCORI
- Engagement as a Path to Rigorous Research
- Break
- PCORI’s Paths to Prioritizing Research Topics
- Advancing PCOR: A Systems Approach
- Break
- PCORI Funding Opportunities
- Wrap-Up and Adjourn
More Succinctly:

- Invite you to consider PCORI research funding
- Consider patient engagement in Research, compare notes
- Solicit the questions that you encounter in doing systems improvement

About PCORI
About PCORI

- An independent, non-profit organization authorized by Congress in 2010
- Funds rigorous CER that answers patients’ questions
- Continuously seeks input from a broad range of stakeholders to guide its work

Mission

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
PCORI’s Board of Governors

Developing and Advancing the Science and Methodology of Patient-Centered Outcomes Research
Major 2012 Accomplishments

- Defined PCOR
- Issued National Priorities/Research Agenda
- Funded 50 Pilot Projects
- Issued primary research funding announcements
- Authorized targeted funding announcements
- Adopted methods standards
- Authorized advisory panels
- Ongoing engagement events

Patient-Centered Outcomes Research Defined

Patient-Centered Outcomes Research (PCOR) helps people and their caregivers make informed health care decisions, allowing their voices to be heard in assessing the value of health care options.
Patient-Centered Outcomes Research Defined

Answers patient-centered questions such as:

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 potential benefits and harms of those options?”
3. “What can I do to improve the outcomes that are most important to me?”
4. “How can clinicians and the care delivery systems they work in help me make the best decisions about my health and healthcare?”

Methodology Standards and Report

- Use of rigorous methods is essential to building trust in PCOR findings
- Methodology Committee charged with defining standards and a translation table to guide best practices for conducting PCOR
- Standards (just adopted) and report (under revision) will be a resource for the entire healthcare community, refined and expanded over time with stakeholder input
Establishing Our Research Agenda

Future priorities and agendas will be informed by the development process, ongoing stakeholder engagement, and PCORI's research results.

National Priorities for Research

- Assessment of Prevention, Diagnosis, and Treatment Options
- Improving Healthcare Systems
- Communication & Dissemination Research
- Addressing Disparities
- Accelerating PCOR and Methodological Research
Engagement as a Path to Rigorous Research

Sue Sheridan, MBA, MIM
Director of Patient Engagement, PCORI

Get Involved
Imperatives

- To invite and utilize wisdom and experience of patients, caregivers, and other stakeholders in our research efforts and eliminate barriers to participation
- Establish a community of trained and informed patients and caregivers as valued partners in all stages of research
- Communicate transparently to stakeholders about our approach to research prioritization, decision making, and funding
- Evaluate and refine engagement processes to continuously incorporate best practices and methods into our work

How to Engage
Engagement Workshops

Transforming Patient-Centered Research: Building Partnerships and Promising Models
- October 27-28, 2012
- Washington, DC

What Should PCORI Study? A Call for Topics from Patients and Stakeholders
- December 4, 2012
- Washington, DC

PCORI Methodology Workshop for Prioritizing Specific Research Topics
- December 5, 2012
- Washington, DC

What Roles Should Patients and Other Stakeholders Play on Research Teams?

Engagement should include:
- Participate in formulating research questions
- Defining essential characteristics of study participants, comparators and outcomes
- Monitoring study conduct and progress
- Disseminating research results
Engagement Principles:
From Thought to Action

- Trust
- Transparency
- Co-learning
- Reciprocal relationships
- Partnerships
- Honesty

Ideal Practice:
Engage “Early and Often”

- Pre-Engagement with Target Population
- Training: Patient and Participant
- Longitudinal Relationship Building/Maintenance
- Require Elements for Proper Indexing – Permit Evaluation
- Evaluation of Engagement over Time


Emerging Model of Engagement

Discussion

PCORI’s Paths to Prioritizing Research Topics

Joe Selby, MD, MPH
Executive Director, PCORI

This speaker has nothing to disclose
PCORI’s Two Paths to “Getting Specific” in Research Funding

Research Criteria Informed by Law
PCORI’s Two Paths to “Getting Specific” in Research Funding

Figure 2: Unique Process

Patient/Stakeholder-Led Approach

- 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 Research Portfolio answering key questions for patients and clinicians

Building on the Existing Evidence Base and Prior Experience

Existing Scientific Work and Literature

Methodology Committee and Methodology Report

Experience of Other Agencies
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 gaps
- Future research needs

**Gap Confirmation**

(in collaboration with AHRQ)

**Research Prioritization**

(multi-stakeholder advisory panels)

**Final Selection**

specific PFAs

(PCORI board)

---

**Phase 1: Topic Generation**

**Process Begins with Patients and Stakeholders**

- Web page
- Social media/marketing
- In-person workshops, focus groups
- Topic generation discussed at patient and stakeholder workshops in the fall
- AHRQ: Future Research Needs (FRNs) reports, systematic gap review
- Other guidelines development processes (such as NQF)
- Gaps identified by NIH, other funding agencies
- Building on existing work or other organizations, prioritization exercises (eg, IOM 100)

**PCORI “Filter”—All Nominated Topics Must:**

- Answer a clinical question around a health care decision
- Be comparative
- Not be related to cost/cost effectiveness
- Align with at least one of PCORI’s national priorities for research

---

12/9/2012
Suggest a Research Question

We want to know what health care question you may be facing

Your input can help us refine our research agenda

www.pcori.org/questions

Phase 2: Gap Confirmation

Topics Provided to AHRQ for Gap Confirmation

Potential Determinations

AHRQ Topic Review and Disposition

Evidence Synthesis Needed

New Primary Research Needed

Answer already known, or research underway

Next Steps

Research Prioritization

Dissemination

In collaboration with AHRQ; contract under development
Phase 3: Research Prioritization

Advisory Panels
Patients and Stakeholders

- Prevention, Diagnosis, Treatment Options
- Communication and Dissemination
- Disparities
- Improving Health Care Systems
- Infrastructure and Methods
- Rare Diseases

Prioritized List of Topics

- 1
- 2
- 3
- 4
- 5
- 6
- 7
- 8
- 9
- 10

Selected From Prioritized List

- 11
- 12
- 13
- 14
- 15
- 16
- 17
- 18
- 19
- 20

Creation of PFAs

Five Prioritization Criteria Adapted from PCORI Funding Criteria

1. Potential for improvement:
   Do preliminary data suggest the research findings may challenge conventional wisdom?
   Will the study reduce the uncertainty around the effect of the interventions?
   How likely are the findings to change practice?
   How long will the information be valid?

2. Patient centeredness – is this a question of importance to patients

3. Impact of the condition on the health of individuals and populations (prevalence, incidence, and other measures of burden of disease)

4. Potential for impact on health care performance

5. Potential for inclusiveness of different populations
Piloting the Process with Patients and Stakeholders

1. Patients and stakeholders participated in a pilot prioritization exercise using topic briefs and online tools. Participation will be by open application.

2. The pilot group convened via several teleconferences to discuss topic briefs and results of initial ranking exercises.

3. The pilot group met in person to reach consensus on a final prioritized list, and to provide feedback to PCORI. The group presented at the December 5th workshop.

4. The revised process will be used by Advisory Panels in Winter 2013 with topics generated from patients and stakeholders across the country.

Summarizing PCORI’s Unique Approach to Prioritization

• Patients and stakeholders engaged in each step of a transparent process:
  ▪ Patients and stakeholders involved in developing process, providing feedback, and members of the future Advisory Panels

• PCORI criteria for research prioritization:
  ▪ Criteria developed to achieve research that will improve patient’s health outcomes, is impactful, and has a high probability of changing clinical practice

• Transparency/visibility embedded in the process:
  ▪ Process shared with public for input and comment
  ▪ Pilot group open to applications
  ▪ Research prioritization methods workshop December 5th broadcast via webcast
Advancing PCOR: A Systems Approach

Chad Boult, MD, MPH, MBA
Director, Healthcare Systems Improvement Program, PCORI

Healthcare Systems Improvement Program

Goal:
To support research that provides new information that patients and healthcare systems use to make decisions that lead to better patient-centered outcomes
How It Works

Structure includes HIT, workforce deployment
Function includes clinical programs, sharing of information, use of decision support tools, pathways, QI processes, internal incentives
Environment includes payment strategies, market forces

Selecting Questions to be Answered

Questions submitted by stakeholders → Ranking by Advisory Panel and Board of Governors → PCORI funding announcement

Stakeholders include patients, families, providers, organization leaders
Criteria: burden, potential to improve practice, potential to improve outcomes, patient-centeredness, inclusiveness
Selecting Applications to be Funded

LOI: standard or large project
Phase 1 review by scientists using 8 criteria
Phase 2 review by scientists, patients and stakeholders using three criteria
Decision by Board of Governors seeks a balance of populations and conditions to be researched

Institute for Healthcare Improvement

Funding Opportunities

Joe Selby, MD, MPH
Executive Director, PCORI
PCORI Pilot Projects Program

PCORI Pilot Projects – 50 Contracts, $31 million

Methods for engaging patients and stakeholders in:

1. Informing PCORI’s national priorities
2. The research process, along with other stakeholders
3. Developing evidence-based systems approaches and decision support tools that account for patient preferences
4. Developing patient-centered outcomes instruments
5. Researching behaviors, lifestyles, and choices
6. Studying patient care team interactions in situations where multiple options exist
7. Analytical methods for CER
PCORI Funding Announcements (PFAs)

- Broad announcements in each priority area
- Emphasize outcomes that matter to patients
- Require patient and stakeholder partnerships
- Must assess dissemination opportunities/challenges
- Requirements for data reproducibility

www.pcori.org/funding-opportunities/
  funding-announcements

Current Funding Opportunities

- PFA: Accelerating Patient-Centered Outcomes Research and Methodological Research
  - Released November 16
  - LOI Deadline: January 31, 2013
- PCORI Challenge: Develop a Patient/Research “Matching” System
  - Launching this month
- Standing PFA’s, each priority area, Cycle III Key Dates:
Discussion

Wrap Up

Joe Selby, MD, MPH
Executive Director, PCORI

This presenter has nothing to disclose
Connect with PCORI

- Updates on funding and other opportunities: www.pcori.org
- Follow @PCORI
- Go to our YouTube channel
- Join our mailing list: www.pcori.org/subscribe