



Recap of Day 1: Address Questions or Concerns

Sue Sheridan, MBA, MIM

Director, Patient Engagement

Advisory Panel Kickoff & Training

April 20, 2013

Patient-Centered Outcomes Research Institute

Agenda for Today

Session	Objective
Recap of Day 1: Address Questions or Concerns	<ul style="list-style-type: none"> ➤ Discuss remaining issues from Day 1 ➤ Quick overview of activities for Day 2
Identifying, Selecting, and Prioritizing Research Questions	<ul style="list-style-type: none"> ➤ Intro to the PCORI Methodology standards ➤ Discuss research prioritization and selection
Reviewing Research Proposals for Funding	<ul style="list-style-type: none"> ➤ Present how patients are involved in PCORI's review of research proposals ➤ Go over criteria for evaluation of proposals' engagement elements
Disseminating Research to the Community	<ul style="list-style-type: none"> ➤ Discuss best practices in disseminating health information to the community ➤ Highlight examples from the field
Discussion of the PCORI Engagement Awards	<ul style="list-style-type: none"> ➤ Gain feedback on Engagement Awards and how to improve the program
Discussion of the Ambassadors Program	<ul style="list-style-type: none"> ➤ Gain feedback on Ambassadors Program and how to improve the program
Defining Patient-Centeredness in Research	<ul style="list-style-type: none"> ➤ Discuss defining relevant terms in the field
Conclusion and Next Steps	<ul style="list-style-type: none"> ➤ Finalize work plan, pledge, and discuss meetings for upcoming year ➤ Consider strategy to identify chairperson

Objectives for the Day

- Feedback on discussion regarding methodology, and patient engagement in prioritization
- Feedback on patient engagement in merit review
- Feedback on disseminating research findings to the public
- Feedback on the PCORI Engagement Awards
- Feedback on the PCORI Ambassadors Program
- Feedback on what “patient centeredness” means to you

Revisit Vision statement

- We the Advisory Panel on Patient Engagement envision a culture of research in which patients, researchers, and clinicians are natural partners with aligned incentives; patients and clinicians are partners in informed medical decision making.



- Ensure meaningful engagement between patients, researchers, clinicians and other stakeholders as equal partners in facilitating the conduct and dissemination of high-value, high-quality patient-centered outcomes research, that has the potential to address patient needs and interests and transform how research is done.

Patient and Stakeholder Engagement in Patient-Centered Outcomes Research

Advise Us as to What PCORI Should Study:

What questions are most important?
(research prioritization)
What outcomes should be studied?
(topic generation)

Review Proposals and Partner in Research

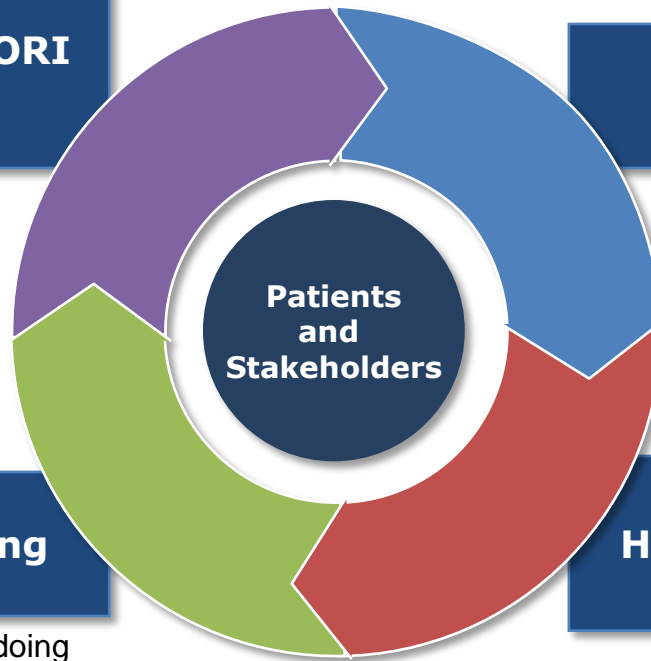
Review research proposals for impact
and patient-centeredness
Participate in conducting research

Tell Us How We Are Doing

How can we improve on what we are doing
and how we are doing it?

Help Us Share the Findings

How do we best communicate
important research findings?





Identifying, Selecting, and Prioritizing Research Questions

Clyde W. Yancy, MD, MSc, FACC, FAHA, MACP

PCORI Methodology Committee

Advisory Panel Kickoff & Training

April 20, 2013

Patient-Centered Outcomes Research Institute

“Update on PCORI: patient engagement as a means to accomplish meaningful CER”

Clyde W. Yancy, MD, MSc, FACC, FAHA, MACP
Magerstadt Professor of Medicine
Chief of Cardiology
Northwestern University, Feinberg School of Medicine
&
Associate Medical Director
Bluhm Cardiovascular Institute
Chicago, IL
cyancy@nmff.org

DISCLOSURES

- Consultant/speaker/honoraria: none
- Editorial Boards: American Heart Journal, American Journal of Cardiology (*associate editor*); Circulation; Circulation-Heart Failure; Circulation- Quality Outcomes; Congestive Heart Failure
- Guideline writing committees: Chair, ACC/AHA, chronic HF; member, hypertrophic cardiomyopathy; member, ACC/AHA Guideline Taskforce, chair, methodology subcommittee
- Federal appointments: FDA: Chair, Cardiovascular Device Panel; ad hoc consultant; NIH CICS study section; advisory committee to the Director; AHRQ- adhoc study section chair; NHLBI- consultant; PCORI- methodology committee member
- Volunteer Appointments: American Heart Association- President, American Heart Association, 2009-2010; American College of Cardiology, Founder- CREDO

Board of Governors Composition

MEMBER	TITLE
Eugene Washington, MD, MSc (Chair)	Vice Chancellor of UCLA Health Sciences, Dean of David Geffen School of Medicine at UCLA
Steven Lipstein, MHA (Vice Chair)	President and Chief Executive Officer of BJC HealthCare
Debra Barksdale, PhD, RN	Associate Professor at the University of North Carolina (UNC) at Chapel Hill School of Nursing
Kerry Barnett, JD	Executive Vice President, Corporate Services, Chief Legal Officer, and Ethics and Compliance Officer, The Regence Group
Lawrence Becker	Director of Strategic Partnerships and Alliances for Xerox Corporation
Carolyn Clancy, MD	Director of the Agency for Healthcare Research and Quality
Francis Collins, MD, PhD	Director of the National Institutes of Health
Leah Hole-Curry, JD	Program Director for the Health Technology Assessment (HTA) program of the Washington State Health Care Authority
Allen Douma, MD	CEO, Empower, LLC, and a member of the AARP Board of Directors
Arnold Epstein, MD	John H. Foster Professor & Chair of the Department of Health Policy and Management at Harvard University
Christine Goertz, DC, PhD	Vice Chancellor, Research and Health Policy, Palmer College of Chiropractic
Gail Hunt	President and CEO of the National Alliance for Caregiving
Robert Jesse, MD, PhD	Principal Deputy Under Secretary for Health, Department of Veterans Affairs
Harlan Krumholz, MD	Harold H. Hines, Jr. Professor of Medicine and Epidemiology & Public Health at Yale University School of Medicine
Richard E. Kuntz, MD, MSc	Senior Vice President and Chief Scientific, Clinical, and Regulatory Officer of Medtronic, Inc.
Sharon Levine, MD	Associate Executive Director for The Permanente Medical Group of Northern California
Freda Lewis-Hall, MD	Executive Vice President and Chief Medical Officer for Pfizer Inc
Grayson Norquist, MD, MSPH	Chair, Dept. of Psychiatry and Human Behavior, Univ. of Mississippi Medical Center
Ellen Sigal, PhD	Chair and founder of Friends of Cancer Research
Harlan Weisman, MD	Chief Science and Technology Officer, Medical Devices and Diagnostics, for Johnson & Johnson
Robert Zwolak, MD, PhD	Vascular Surgeon at Dartmouth-Hitchcock Medical Center

PCORI Origin & Appointments



On March 23, 2010, the 111th Congress created PCORI as part of the Patient Protection and Affordable Care Act (Public Law 111–148)



Board of Governors

Press Release (September 23, 2010)

WASHINGTON, DC– Gene L. Dodaro, Acting Comptroller General of the United States and head of the U.S. Government Accountability Office (GAO), today announced the appointment of 19 members to the Board of Governors for the new Patient-Centered Outcomes Research Institute (PCORI).

Methodology Committee

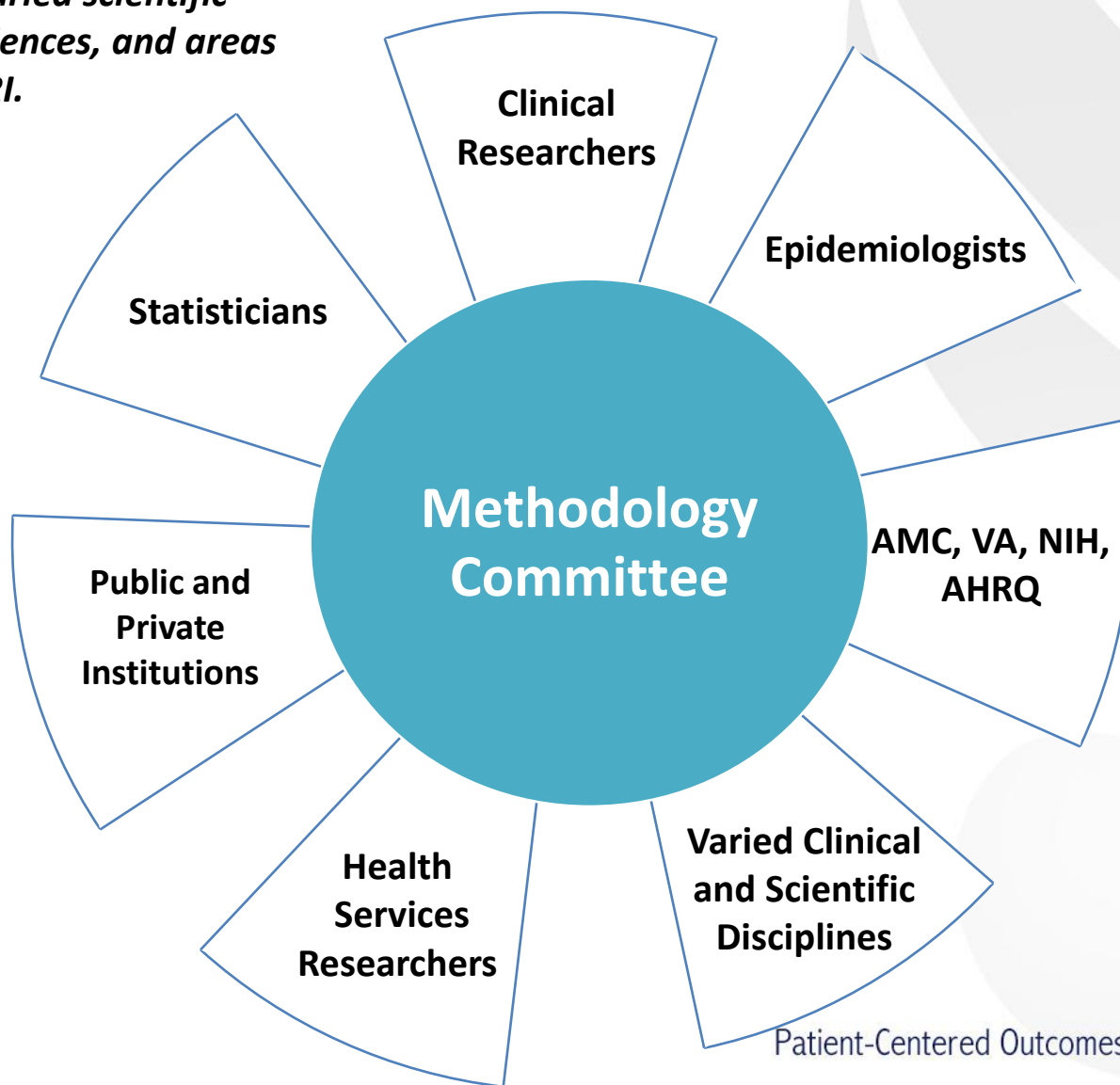
Press Release (January 21, 2011)

WASHINGTON, DC — Gene L. Dodaro, Comptroller General of the United States and head of the U.S. Government Accountability Office (GAO), today announced the appointment of 15 members to the Methodology Committee of the Patient-Centered Outcomes Research Institute (PCORI).



Methodology Committee Composition

The 17 member Methodology Committee brings varied scientific backgrounds, experiences, and areas of expertise to PCORI.



Methodology Committee Composition

MEMBER	TITLE
Sherine Gabriel, MD, MSc (Chair)	Professor of Medicine and of Epidemiology , William J. and Charles H. Mayo Professor at Mayo Clinic
Sharon-Lise Normand, MSc, PhD (Vice Chair)	Professor of Health Care Policy (Biostatistics) in the Department of Health Care Policy at Harvard Medical School and Professor in the Department of Biostatistics at the Harvard School of Public Health
Naomi Aronson, PhD	Executive Director of the Blue Cross and Blue Shield Association Technology Evaluation Center
Ethan Basch, MD, MSc	Associate Attending Physician and Outcomes Scientist at Memorial Sloan-Kettering Cancer Center
Alfred Berg, MD, MPH	Professor in the Department of Family Medicine at the University of Washington in Seattle
David Flum, MD, MPH	Professor in the Department of Surgery and Adjunct Professor in Health Services and Pharmacy at the University of Washington Schools of Medicine, Public Health and Pharmacy
Steven Goodman, MD, PhD	Associate Dean for Clinical and Translational Research, School of Medicine , Stanford University
Mark Helfand, MD, MS, MPH	Professor of Medicine and Professor of Medical Informatics and Clinical Epidemiology at the Oregon Health & Science University
John Ioannidis, MD, DSc	C.F. Rehnborg Chair in Disease Prevention, Professor of Medicine, Professor of Health Research and Policy, and Director of the Stanford Prevention Research Center at Stanford University
Michael Lauer, MD	Director of the Division of Cardiovascular Sciences at the National Heart, Lung, and Blood Institute
David Meltzer, MD, PhD	Chief of the Section of Hospital Medicine, The University of Chicago
Brian Mittman, PhD	Director, VA Center for Implementation Practice and Research Support, Department of Veterans Affairs Greater Los Angeles VA Healthcare System
Robin Newhouse, PhD, RN	Assistant Dean for the Doctor of Nursing Practice Program and Associate Professor, Organizational Systems and Adult Health, University of Maryland School of Nursing
Sebastian Schneeweiss, MD, ScD	Associate Professor of Medicine and Epidemiology at Harvard Medical School and Vice Chief of the Division of Pharmacoepidemiology and Pharmacoeconomics at the Brigham and Women's Hospital
Jean Slutsky, PA, MSPH	Director of the Center for Outcomes and Evidence , Agency for Healthcare Research and Quality
Mary Tinetti, MD	Gladdys Phillips Crofoot Professor of Medicine, Epidemiology, and Public Health in the Division of Geriatrics at Yale University School of Medicine
Clyde Yancy, MD, MSc	Professor of Medicine, Chief, Cardiology, Northwestern University Feinberg School of Medicine

PCORI Mission Statement

The 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 is an **independent, non-profit** organization authorized by Congress committed to continuously seeking input from patients and a broad range of stakeholders to guide its work.*



Patient-Centered Outcomes Research Institute

Taking Patient-Centeredness Seriously



Understanding
the choices
patients face



Aligning research questions
and methods with
patient needs



Providing patients and
providers with information
for better decisions

PCOR Definition

Defining Patient-Centered Outcomes Research (PCOR)

Helps people and their caregivers communicate and make informed health care decisions, allowing their voices to be heard in assessing the value of health care options. This research answers patient-centered questions such as:

Expectations	Options	Outcomes	Decisions
“Given my personal characteristics, conditions and preferences, what should I expect will happen to me?”	“What are my options and what are the potential benefits and harms of those options?”	“What can I do to improve the outcomes that are most important to me?”	“How can clinicians and the care delivery systems help me make the best decisions about my health and healthcare?”

PCOR Definition

In order to answer these patient-focused questions, PCOR:

- Assesses the benefits and harms of preventive, diagnostic, therapeutic, palliative, 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, resource availability, and other stakeholder perspectives.

National Priorities for Research and Research Agenda

Assessment of Options for Prevention, Diagnosis, and Treatment

- ✓ • Comparisons of alternative clinical options to support personalized decision-making and self-care
- Identifying patient differences in response to therapy
- Studies of patient preferences for various outcomes

Improving Healthcare Systems

- ✓ • Improving support of patient self-management
- Focusing on coordination of care for complex conditions and improving 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
- Methods for training researchers, patients to participate in PCOR
- Establishing methodology for the study of rare diseases

Criteria for Research Outlined by Law

**Impact on Health of
Individuals and
Populations**

**Addresses
Current Gaps in
Knowledge/
Variation in Care**

**Patient-
Centeredness**

**Improvability
through Research**

**Impact on Health
Care System
Performance**

**Rigorous Research
Methods**

**Inclusiveness of
Different
Populations**

**Potential to
Influence Decision-
Making**

**Efficient Use of
Research
Resources**



*“This is going to be
research done
differently!”*

PCORI Board Member **Harlan Krumholz, MD**
National Patient and Stakeholder Dialogue
National Press Club, Washington, DC
February 27, 2012

What Makes PCORI Funding Different?

- Special features include:
 - Patient & Stakeholder Engagement Plan
 - Dissemination and Implementation Assessment
 - Reproducible and Transparent Research Plan
 - PCORI Criteria Outlined by Statute
 - Complies with Methodology Standards
 - User-friendly announcements to encourage broader range of applicants



Stakeholder Engagement in PCORI-funded Research

- Key stakeholders are engaged early and throughout the research process.
- PCORI will score applications on how meaningfully patients and stakeholders are engaged.
- Key stakeholders include those for whom the results of the research will be relevant:

Patients, Caregivers, Consumers and organizations representing them
Researchers/Research Associations
Clinicians/Clinician Associations
(Physicians, Nurses, Pharmacists, Professional Societies/Associations, and Other Clinicians)

Organizational Providers (Hospitals, Integrated Delivery Systems, Clinics, Community Health Centers, Pharmacies, Nursing Facilities)

Purchasers (Employers, Self-Insured, Government and Other Entities)

Payers (Insurers, Medicare and Medicaid, States and Labor Trusts)

Industry (Drug, Device, Biotechnology, EHR Vendors)

What roles should patients and stakeholders play in research teams?

The engagement of patients and stakeholders should include:

- Participation in formulation of research questions;
- Defining essential characteristics of study participants, comparators, and outcomes;
- Monitoring of study conduct and progress; and
- Dissemination of research results.

Patient and Stakeholder Engagement 2012

- Building communities of patients and stakeholders – using website, social media, face-to-face meetings
- Strengthening ties with advocacy associations, professional clinician organizations, purchaser organizations, research community
- Refining the PCORI Research Agenda
 - Convening multi-stakeholder workshops focused on each of the National Priorities
 - Forming multi-stakeholder Advisory panels
 - Using social media, surveys to obtain broad input

The PCORI Methodology Committee



Methodology Committee Charge

The Methodology Committee is charged with making **recommendations** regarding methods for patient-centered outcomes, which includes:

- **guidance** about **the appropriate use of methods** in such research
- **establishing priorities to address gaps** in research methods or their application

Methodology Committee Role

Vision

PCORI methodological knowledge and standards are widely adopted as best practices across the PCORI stakeholder community.

Mission

To become the “go to” PCOR scientific methodology resource and the "how to" group for PCORI—addressing methodological areas of focus, advancing methodological science and, thereby, enabling PCORI to accomplish its agenda.

Scientific Advisor to the Board

The Methodology Committee also serves as scientific advisor to the Board regarding research, dissemination, infrastructure and capacity building as well as patient and stakeholder engagement



The **First** Methodology Committee Report



Patient-Centered Outcomes Research Institute

Draft Methodology Report:
“Our Questions, Our Decisions: Standards for
Patient-centered Outcomes Research”

PCORI Methodology Committee

Mark Helfand, Alfred Berg, David Flum, Sherine Gabriel,
and Sharon-Lise Normand, *Editors*

Published for Public Comment July 23, 2012

Methodology Report



Patient-Centered Outcomes Research Institute

Draft Methodology Report:
"Our Questions, Our Decisions: Standards for
Patient-centered Outcomes Research"

PCORI Methodology Committee

Mark Helfand, Alfred Berg, David Flum, Sherine Gabriel,
and Sharon-Lise Normand, *Editors*

Published for Public Comment July 23, 2012

- | | |
|-------------------|--|
| Chapter 1. | Introduction |
| Chapter 2. | How the Methodology Committee Developed the Recommended Standards |
| Chapter 3. | Overview of the Standards |
| Chapter 4. | Methodological Standards for Patient-Centeredness of Research Proposals and Protocols |
| Chapter 5. | Methods for Prioritizing Patient-Centered Outcomes Research |
| Chapter 6. | Choosing Data Sources, Research Design, and Analysis Plan: Translation Framework and Development of a Translation Table |
| Chapter 7. | General and Cross-Cutting Research Methods |
| Chapter 8. | Design-Specific Methods |
| Chapter 9. | Next Steps |



Patient-Centered Outcomes Research Institute

NEJM Article and Ads

Digital Ads released in *Annals of Internal Medicine*; *Science Translational Medicine*; *JAMA*; *NEJM*; *Nature*; and *Health Affairs*



Help Shape Patient-Centered
Research Methods

[CLICK HERE](#)

HEALTH
AFFAIRS

*The Policy Journal
of the Health Sphere*



The NEW ENGLAND
JOURNAL of MEDICINE

[HOME](#)ARTICLES & MULTIMEDIA ^w

ISSUES ▾

SPECIALTIES & TOPICS ▾

FOR AUTHORS

CME »



Perspective

Getting the Methods Right — The Foundation of Patient-Centered Outcomes Research

Sherine E. Gabriel, M.D., and Sharon-Lise T. Normand, Ph.D.

N Engl J Med 2012; 367:787-790 | August 30, 2012

Article

References

Health care in the United States has changed dramatically over the past several decades. Today, patients have more options than ever. Making the right choices, whether for prevention, diagnosis, or treatment, requires a critical appraisal of the potential benefits and harms of the options, within the context of the patient's characteristics, conditions, and preferences.

Many of these choices are available thanks to advances in medical research. Yet most patients and many clinicians find research somewhat mysterious. They have difficulty sorting through the mountains of medical evidence to identify information that is reliable and actionable for their unique circumstances. Patient-centered outcomes research and comparative-effectiveness research promise to enhance decision makers' ability to fully understand and weigh alternatives. But just as health care interventions and delivery strategies have advanced markedly in recent decades, so have

Methods

Selected Milestones in Health Care Interventions and Delivery Strategies and in Research Methods.*

Decade	Milestones in Health Care Interventions and Delivery Strategies	Milestones in Research Methods
1940s	Antibiotic agents (penicillin and streptomycin), kidney dialysis, general anesthesia, radiotherapy, first heart-pump machine, influenza vaccine, Papanicolaou (Pap) smear to detect cervical cancer, cortisone, intraocular lens implants for cataracts	First large-scale, randomized, controlled trial
1950s	Cardiopulmonary resuscitation, kidney transplantation, vaccination against poliomyelitis, chlorpromazine for schizophrenia, Zeiss fluorescence microscope, antitubercular therapy, cardiac pacemaker, artificial heart valve, successful open-heart bypass surgery	Case-control methodology, Kaplan-Meier survival estimator
1960s	Charnley's hip replacement, coronary-artery bypass grafting surgery, heart transplantation, oral contraceptive pill, prenatal diagnosis of Down's syndrome	Explanatory versus pragmatic trial concept, data and safety monitoring, growth of observational research methods committees
1970s	Cure for some childhood cancers; neonatal intensive care; computed tomography; coronary angiography; quality measures in health care; ambulatory surgery; vaccinations against smallpox, measles, mumps, rubella, and pneumonia	Cox proportional-hazards model; meta-analysis; ascendancy of randomized, controlled trials; statistical stopping rules
1980s	Insulin therapies for diabetes mellitus, thrombolysis for heart attacks, anti-hypertensive drugs, magnetic resonance imaging, robotic surgery, permanent artificial-heart implant, deep-brain electrical stimulation system, first laser surgery on the human cornea, hepatitis B vaccine	Propensity score; large, simple trials; prognostic models (e.g., Framingham risk score), growth of decision and cost-effectiveness analyses
1990s	Coronary stents, triple therapy for the acquired immune deficiency syndrome, introduction of biologics, "physician extenders," facial transplantation, vaccine against hepatitis A, first rotavirus vaccines	Evidence-based medicine, cumulative meta-analysis, reporting guidelines (CONSORT statement), ascendancy of registries, electronic health records, Markov chain Monte Carlo sampling for Bayesian inference
2000s	Human Genome Project completed, drug-eluting coronary stents, FDA guidance on patient-reported outcomes, minimally invasive techniques for surgery, human papillomavirus vaccine to prevent cervical cancer	Trial registration (ClinicalTrials.gov), comparative-effectiveness research, implementation science, large-scale genomic research, reproducible research
2010s	Genomics, epigenomics, individualized medicine, health information technology, emergence of telehealth, meaningful-use initiatives, Affordable Care Act becomes law	Patient-centered outcomes research

* Information on health care interventions and delivery strategies are from Le Fanu.¹ CONSORT denotes Consolidated Standards of Reporting Trials, and FDA Food and Drug Administration.

Methodology Report Development

Committee Expertise

Methods Selection

- Working groups identified and prioritized major research methods questions to be addressed

Information Gathering

- Researchers contracted to address selected topics
- Contractors developed research materials (e.g., reports, summary templates for proposed standard)
- MC solicited for external feedback on the translation table (RFI)
- Workshops held to discuss contractor findings, with invited experts in attendance

Internal Review

- MC conducted in-depth internal review of materials developed by contractors, and support staff
- MC independently submitted preliminary votes on proposed standards
- MC deliberated to reach consensus on recommendations to be endorsed in the report

Report Generation

- Refined recommendations and report content per committee evaluations and discussions

Methodology Report – Information Gathering

17 reports* addressing 15 topics, from MC-led contracted research, informed 1st Methodology Report

Topics

1. Design, Conduct, and Evaluation of Adaptive Randomized Clinical Trials
2. Conduct of Registry Studies
3. Design of Patient-Reported Outcomes Measures (PROMS)
4. Use of Collaborative or Distributed Data Networks
5. Prevention and Handling of Missing Data
6. Design, Conduct and Evaluation of Diagnostic Testing
7. Causal Inference Methods in Analyses of Data from Observational and Experimental Studies
8. Addressing Heterogeneity of Treatment Effects: Observational and Experimental PCOR

Methodology Report – Information Gathering

Topics Contracted Research Reports (Cont'd)

9. Involving Patients in Topic Generation
10. Value-of-Information in Research Prioritization
11. Peer Review as a Method for Research Prioritization
12. Examination of Research Gaps in Systematic Reviews for Research Prioritization
13. Integrating Patients' Voices in Study Design Elements with a Focus on Hard-to-Reach Populations
14. Eliciting Patient Perspective
15. PCORI Expert Interviews





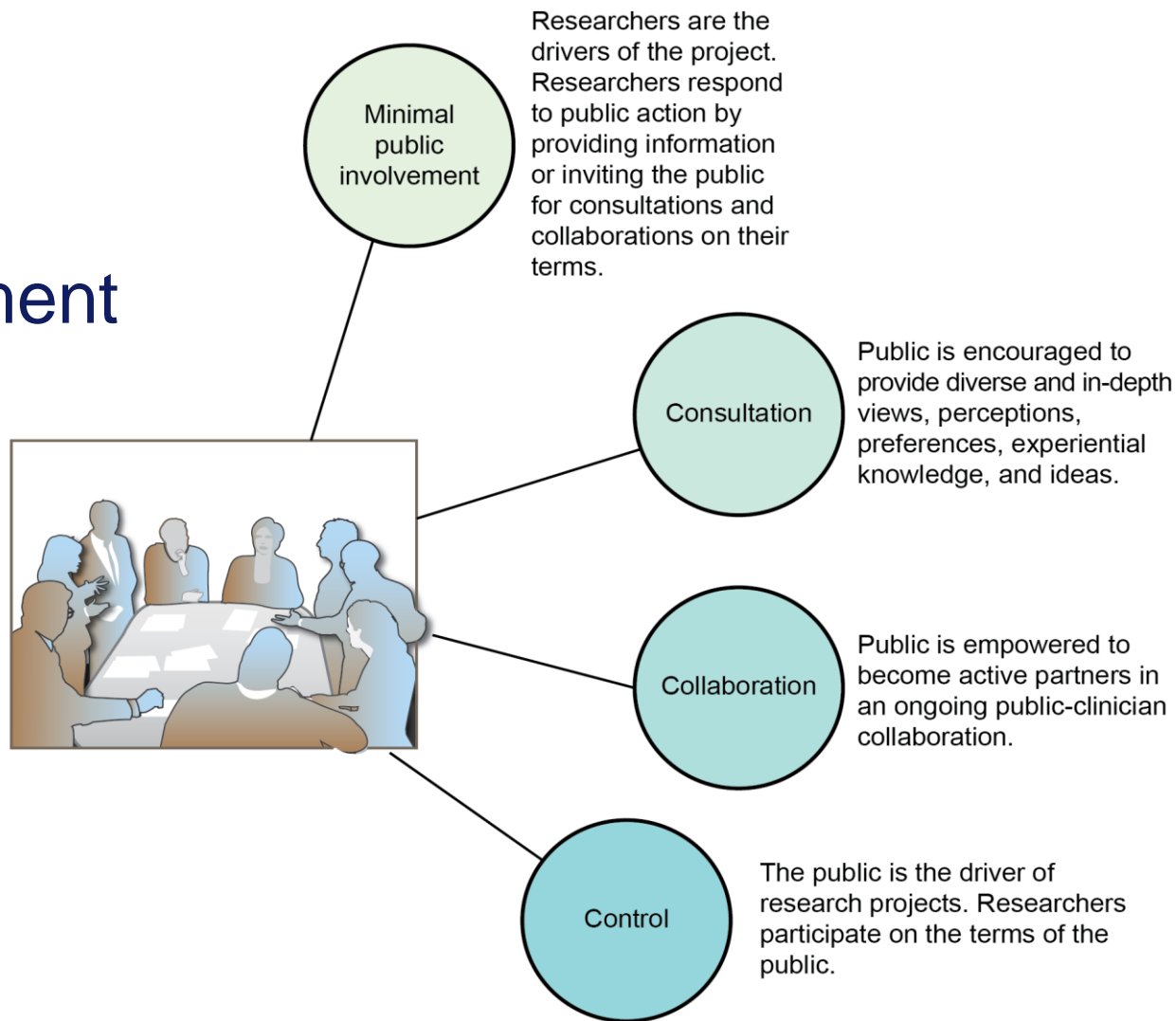
**Methods for Involving
Patients in
Topic Generation for Patient-
Centered Comparative
Effectiveness Research –
An International Perspective**

Patient-Centered Outcomes Research Institute

Project Framework

- Discuss the levels of engagement
- Summarize qualitative research strategies and methods
 - Provide specific examples
- Discuss facilitators of public engagement
- Describe three types of scientific research data as part of the engagement process
- Propose a process of engagement






Levels of Engagement



Public Engagement as Research

- Is the objective study of the individual experience
- Uses mostly qualitative research strategies and methods

Scientific Strategies the Framework of Engagement

-  Phenomenology
-  Ethnography
-  Grounded theory
-  Action research
-  Survey

Methods and Processes

- Interviews (one-on-one or group interviews, photovoice)
- Observation
- Documents
- Questionnaires

Consultation

- Public-physician partnerships

Collaboration

Views, Opinions, Experience as Research Data

- Generates mostly textual data
- That are categorized into themes
- And can be translated into research areas and topics

Case 1

- In-depth one-on-one interviews and focus group interviews
- 40 patients with ulcerative colitis
- Patients identified 9 research areas
- Only during in-depth interview patients asked about prenatal genetic testing for a possible termination of pregnancy if the fetus was affected

Case 2

- Public-Clinician Partnership to develop research topics for urinary incontinence (James Lind Alliance)
 - Lay members and clinicians consult with their peers to include diverse views
 - Systematic reviews are used to generate additional topics and to avoid duplication of research
 - Nominal Group Technique to reach a consensus and prioritize topics

Case 2 continued...

- 8 patient and 13 clinician groups participated
- Final database contained 226 research questions:
 - 79 unique questions from patients
- The group created a “Top 10” list of research questions
- Since then, 5 studies have been funded, 5 new systematic reviews are in progress, 5 questions are under consideration for funding.

Case 3

- Advisory panel to identify research topics and research priorities related to urinary incontinence in women
 - What can researchers study to make your life better?
 - What should we measure to see if your life is better?

Five main research areas emerged:

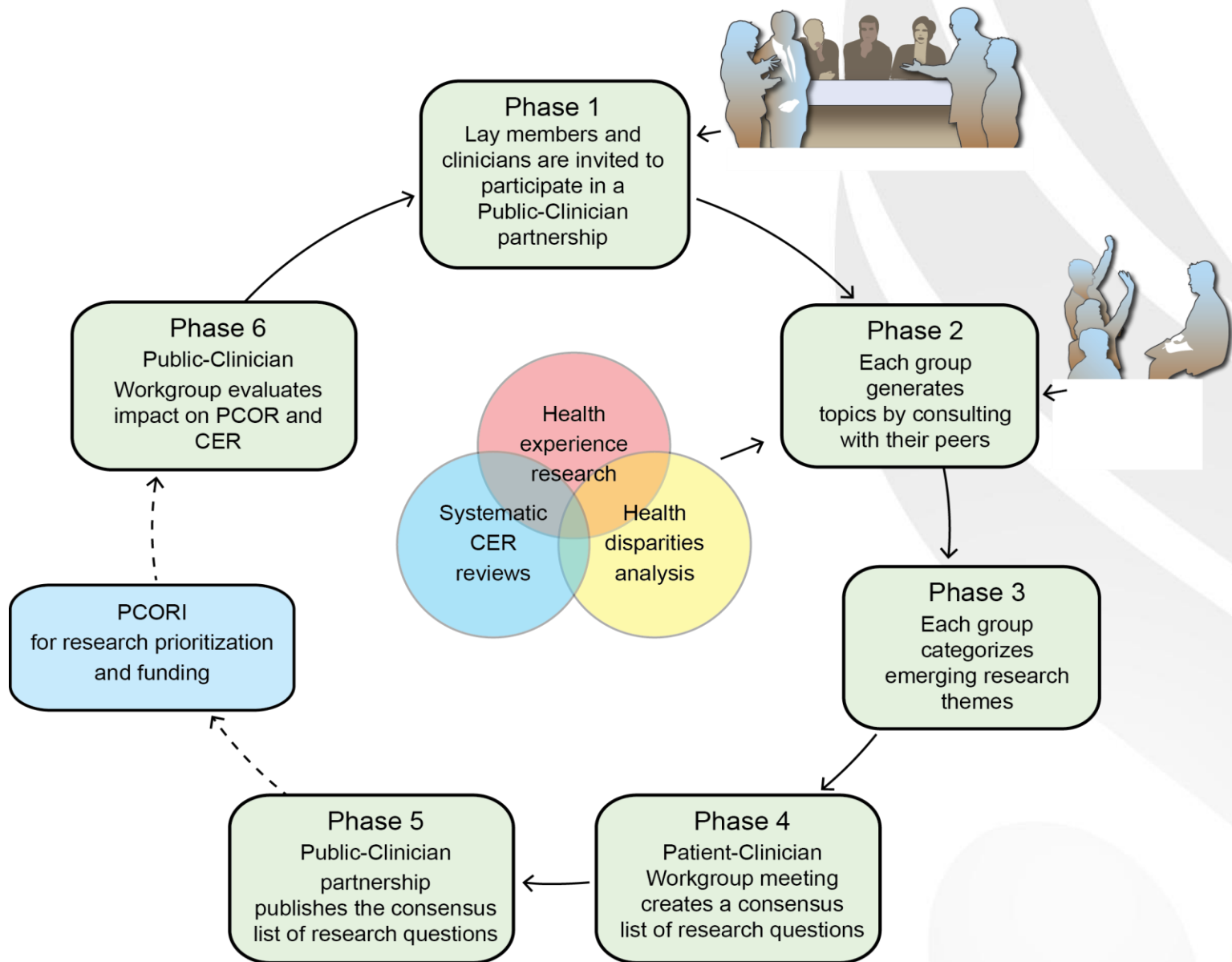
- Interventions that make seeking help easier
- Information giving and interventions designed to make day-to-day life more manageable
- The true costs of incontinence
- Causes
- Effects of lifestyle modification on incontinence
- Patients considered quality of life the most important outcome measures.

Facilitators that Overcome the Barriers to Public Engagement

- Creating a patient-centered organizational structure
- Supporting members of the public
- Communicating clear expectations
- Provide training
- Using processes that give an equal voice to professional and lay participants
- Using a variety of engagement methods

The Role of Scientific Data

- Comparative effectiveness reviews
- Health disparities research
- Health experience research





PCORI Awards

***The first
experience -
2012***

Patient-Centered Outcomes Research Institute



Recommended Funding Slate

4 Addressing disparities
(6% of PFA total)

9 Assessing options
(4% of PFA total)

6 Communication & dissemination
(7% of PFA total)

6 Improving healthcare systems
(6% of PFA total)

25 Total
(5% of total)

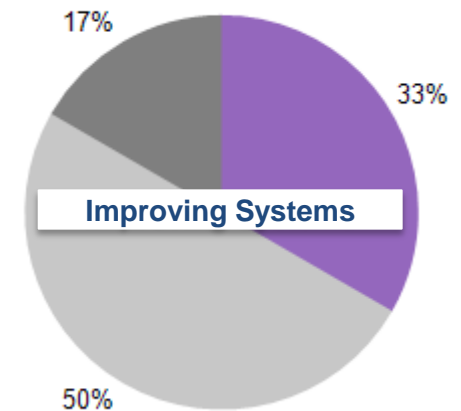
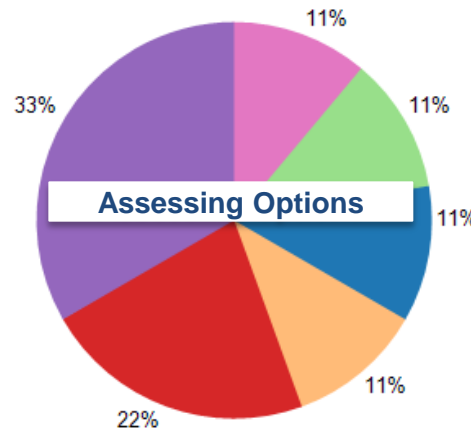
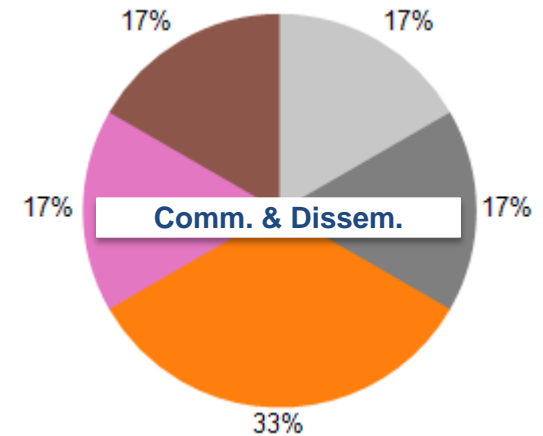
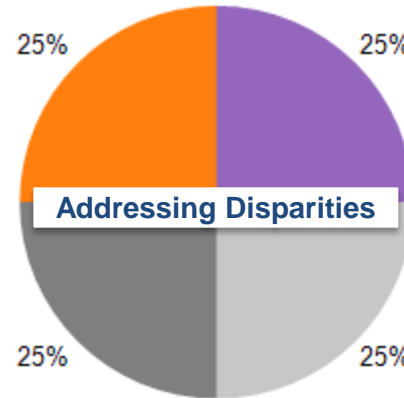
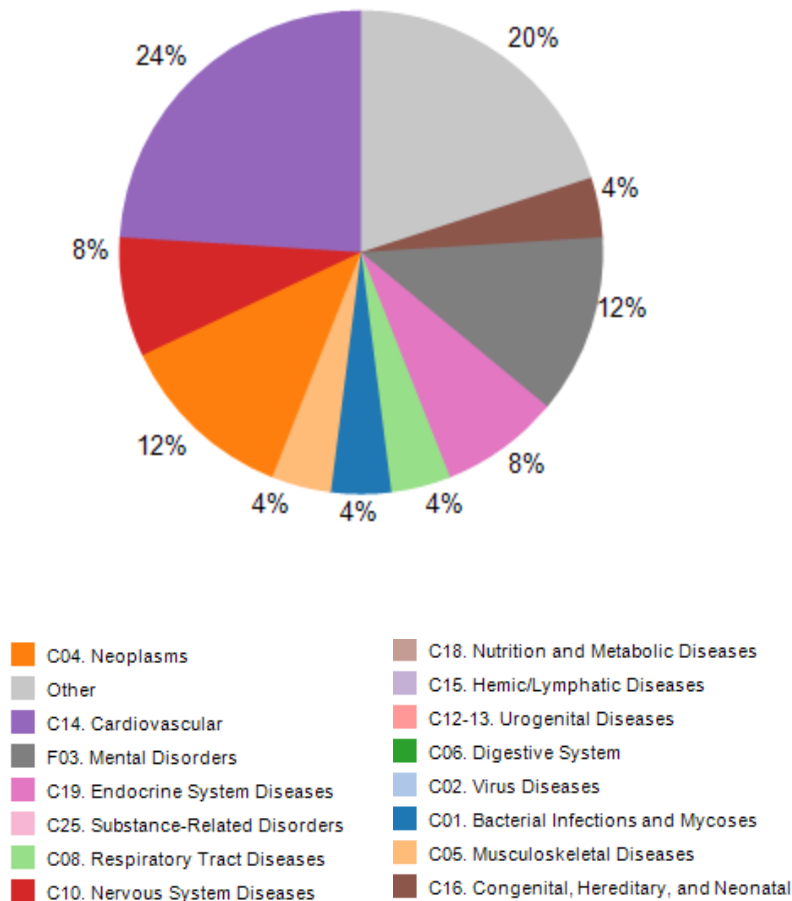
Slate includes all applications scoring 30 or better.

	Average	Total
Addressing Disparities	1,746,223	6,984,893
Assessment of Options	1,698,072	15,282,647
Communication and Dissemination	1,421,132	8,526,789
Improving Healthcare Systems	1,653,590	9,921,541
Grand Total	1,628,635	40,715,870

% of total means of those applications deemed responsive

Recommended Funding Slate

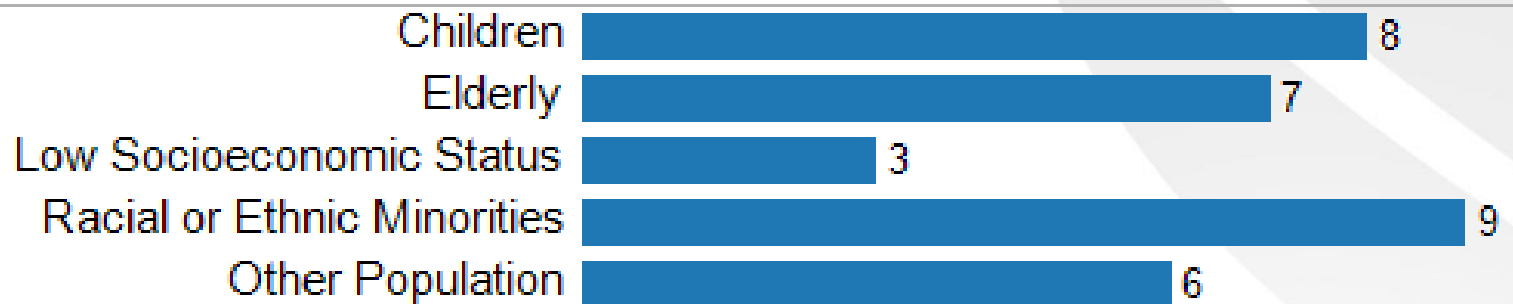
Conditions



"Other" typically indicates a non-condition response to the question. Responses include: insurance coverage, primary care, surgical decision making, clinical management, comprehensive health systems., etc.

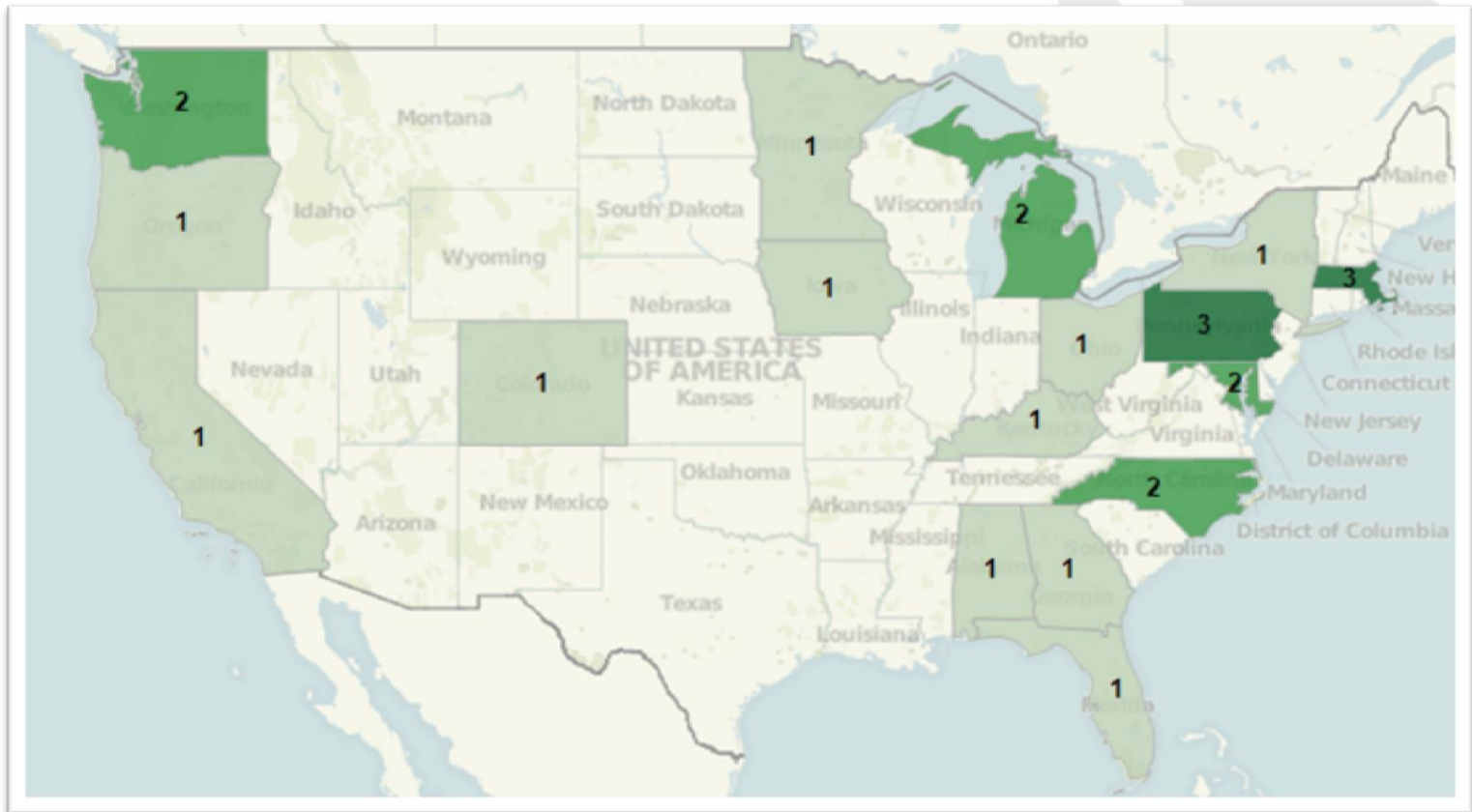
Recommended Funding Slate

Populations Overall



Other Population includes women, disabled persons, and veterans.

Locations



Recommended Funding Slate

Project Titles: Addressing Disparities

1. Cultural tailoring of educational materials to minimize disparities in HPV vaccination
2. Long-term outcomes of community engagement to address depression outcomes disparities
3. Reducing Disparities with Literacy-Adapted Psychosocial Treatments for Chronic Pain: A Comparative Trial
4. Reducing Health Disparities in Appalachians with Multiple Cardiovascular Disease Risk Factors

Recommended Funding Slate

Project Titles: Assessing Options

1. A Comparison of Non-Surgical Treatment Methods for Patients with Lumbar Spinal Stenosis.
2. Cognitive AED Outcomes in Pediatric Localization Related Epilepsy (COPE)
3. Comparative effectiveness of adolescent lipid screening and treatment strategies
4. Comparative Effectiveness of Intravenous v. Oral Antibiotic Therapy for Serious Bacterial Infections
5. Comparative effectiveness of rehabilitation services for survivors of an acute ischemic stroke
6. Evaluation of a Patient-Centered Risk Stratification Method for Improving Primary Care for Back Pain
7. Improving Psychological Distress Among Critical Illness Survivors and Their Informal Caregivers
8. Selection of Peritoneal Dialysis or Hemodialysis for Kidney Failure: Gaining Meaningful Information for Patients and Caregivers
9. Shared Decision Making in the Emergency Department: The Chest Pain Choice Trial

Recommended Funding Slate

Project Titles: Communications & Dissemination

1. Decision Support for Parents Receiving Genetic Information about Child's Rare Disease
2. Extension Connection: Advancing Dementia Care for Rural and Hispanic Populations
3. Patient-Identified Personal Strengths (PIPS) vs. Deficit-Focused Models of Care
4. Presenting Patient-Reported Outcomes Data to Improve Patient and Clinician Understanding and Use
5. Relapsed childhood neuroblastoma as a model for parental end-of-life decision-making
6. Shared Medical Decision Making in Pediatric Diabetes

Recommended Funding Slate

Project Titles: Improving Healthcare Systems

1. Creating a Clinic-Community Liaison Role in Primary Care: Engaging Patients and Community in Health Care Innovation
2. Improving Palliative and End-of-Life Care in Nursing Homes
3. Innovative Methods for Parents And Clinics to Create Tools (IMPACCT) for Kids' Care
4. Optimizing Behavioral Health Homes by Focusing on Outcomes that Matter Most for Adults with Serious Mental Illness
5. Relative patient benefits of a hospital-PCMH collaboration within an ACO to improve care transitions
6. The Family VOICE Study (Value Of Information, Community Support, and Experience): a randomized trial of family navigator services versus usual care for young children treated with antipsychotic medication

PCOR milestones

Metrics	2013 Milestones Outputs Only	2017 Milestones Mainly Outputs Some Instances toward Goal?	2022 Milestones Lots of Outputs Major Progress toward Goal
PCOR studies resulting from both our broad and targeted PFAs	Advisory Panels established ~ 130 studies funded via broad PFAs ~ 40 studies funded via targeted PFAs	Completed Studies ~ 25 studies from 2012 ~ 130 studies from 2013 ~ 40 studies from 2013 targeted Studies Underway ~ 700 studies from 2014–17 broad and targeted PFAs, etc.	Completed Studies ~ 1,200 from 2012–19 High proportion actionable Studies Underway ~ 500 studies from 2020–22 broad and targeted PFAs, etc.
Targeted topic areas to study in depth	Ad hoc work groups established 5 targeted topics underway – fibroids, falls, asthma, obesity, back pain	Number of targeted topics with multiple cycles from 2013 – 16 Number of targeted topics initiated in 2017	Dozens of targeted topics studied in depth through multiple cycles

A Special Thank You to...



Editing Team/ Interim Researchers

Andrew Holtz MPH
Heidi D. Nelson, MD, MPH
Ed Reid, MS, MAT
Annette Totten, PhD
Tim Carey, MD, MPH
Howard Balshem
Justine Siedenfeld
Crystal Smith-Spangler, MD

Workshop External Attendees

Kate Bent, PhD
Karl Claxton, PhD
Christine Laine, MD, MPH, FACP
Richard Nakamura, PhD
Evelyn Whitlock, MD, MPH
Tanisha Carino, PhD
Steve Phurrough, MD, MPA
Cynthia Chauhan, M.S.W.
Pat Deverka, M.D.
Kay Dickersin, M.A., Ph.D
Lorraine Johnson, J.D., M.B.A
David Osoba, B.Sc., M.D.,
F.R.C.P.C
Dennis Revicki, Ph.D.
John Santa, M.D., M.P.H.
Albert Wu, M.D., M.P.H

Principal Investigators and Research Team Members

- University of Maryland, Pharmaceutical Health Services Research Department (Daniel Mullins, Ph.D.)
- Mayo Clinic, Knowledge and Evaluation Research Unit (M. Hassan Murad, M.D., MPH)
- Oregon Health & Science University, The Center for Evidence-Based Policy (Pam Curtis, M.S.)
- Oxford Outcomes, Ltd., Patient Reported Outcomes (Andrew Lloyd, Ph.D.)
- Northwestern University/UNC Chapel Hill (Zeeshan Butt, Ph.D. /Bryce Reeve, Ph.D.)
- Johns Hopkins University (Tianjing Li, MD, MHS, PhD)
- Johns Hopkins University – School of Medicine (Ravi Varadhan, PhD)
- Berry Consultants (Scott Berry)
- Brown University (Constantine Gatsonis, PhD)
- Brigham and Women's hospital and Harvard Medical School (Josh Gagne, PharmD, ScD)
- Outcome Sciences, Inc. (A Quintiles Company) (Richard Giklich, MD)
- University of California San Diego (UCSD) (Lucila Ohno-Machado, MD, PhD)
- Hayes, Inc. (Petra Nass, PhD)
- NORC at the University of Chicago (David Rein, PhD)
- Duke Evidence-Based Practice Center (Evan Myers, MD, MPH)
- Medical College of Wisconsin (Theodore Kotchen, MD)

Electronic Data Systems Interviewees

*57 interviewees from:

- Government
- Associations
- Academia
- Commercial
- Health Care Provides

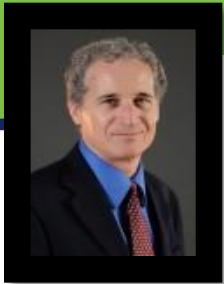
Respondents to RFI — Input Draft Translation Table Framework

*Over 15 submissions received

PCORI Staff
Institute

Interim Consultants

PCORI METHODOLOGY COMMITTEE



Brian Mittman



Al Berg



Ethan Basch



Sherine Gabriel



Jean Slutsky



Michael Lauer



Clyde Yancy



Naomi Aronson



Mary Tinetti



David Flum



Robin Newhouse



Sharon Lise-Normand



David Meltzer



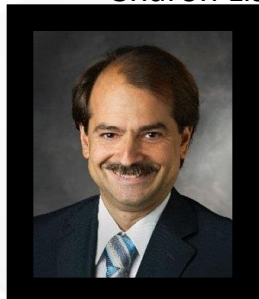
Mark Helfand



Sebastian Schneeweiss



Steven Goodman



John Ioannidis



Thank You!

Patient-Centered Outcomes Research Institute



Reviewing Research Proposals for Funding

Kristen Metzger, MPA, MSCJ

Project Coordinator, Contracts, PCORI

Advisory Panel Kickoff & Training

April 20, 2013

Patient-Centered Outcomes Research Institute



Disseminating Research to the Community

Anne Beal, MD, MPH

*Deputy Executive Director, Chief Operating Officer &
Chief Officer for Engagement, PCORI*

Advisory Panel Kickoff & Training

April 20, 2013

Patient-Centered Outcomes Research Institute

The Basis for a PCORI Blueprint on Dissemination and Implementation

Legislation*:

The purpose of the Institute is to assist patients, clinicians, purchasers, and policy-makers 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 the medical treatments, services, and items described in subsection (a)(2)(B).

Action Plan:

- **Encourage researchers to develop dissemination plan.**
- **Fund research in the Communications and Dissemination Program**
- **Develop PCORI dissemination plan and infrastructure in collaboration with AHRQ**

*Patient Protection and Affordable Care Act, Pub. L. No. 111-148, §2702, 124 Stat. 119, 318-319 (2010).

Establishing a PCORI Blueprint and Framework for Dissemination & Implementation

DISSEMINATION

+

IMPLEMENTATION

PCORI's Blueprint for

Dissemination and Implementation is being established:

- To guide the organization in disseminating the research findings of funded research conducted in the national program areas; and
- To [enhance implementation](#) by actively facilitating how PCORI's research findings can be used by health care decision-makers.
- To [evaluate](#) how the effect of the dissemination of such findings reduces practice variation and disparities in health care.

Touch Points for Effective Dissemination

- Speeding the implementation of and use of PCOR
- Becoming a trusted resource for information
- Engaging stakeholders from across the healthcare community to include PCORI research in training, practice, and standards

[pre-award]

Topic Generation:
Likelihood of
Implementation in
Practice

Proposals:
Researchers
Proactively Plan to
Disseminate

[post-award]

Patients and
Stakeholders Engaged
in the Research Study

Staff Actively Evaluates
Progress

Share Early Results

[completed]

Researchers Carry Out
Dissemination Plans

Patients and
Stakeholders Engaged
in the Process

Mediums of Dissemination

- White Papers
- Manuscripts
- Publishing in Journals and Scientific Publications
- Workshops
- Media Coverage and Press Release
- Research Summary Documents
- Flyers, Posters, Brochures and Research Briefs
- Policy Briefs
- Study Newsletters
- Community Agency Publications and Websites and List-serves
- Local Events, Seminars, Conferences, Community Meetings and Workshops
- Letter of Thanks to Study Participants
- Guidelines and Standards of Care
- Op-Eds
- Others?

Questions

 Thank you for your time and attention!



Discussion of PCORI Engagement Awards

Suzanne Schrandt, JD

Deputy Director, Engagement, PCORI

Advisory Panel Kickoff & Training

April 20, 2013

Patient-Centered Outcomes Research Institute

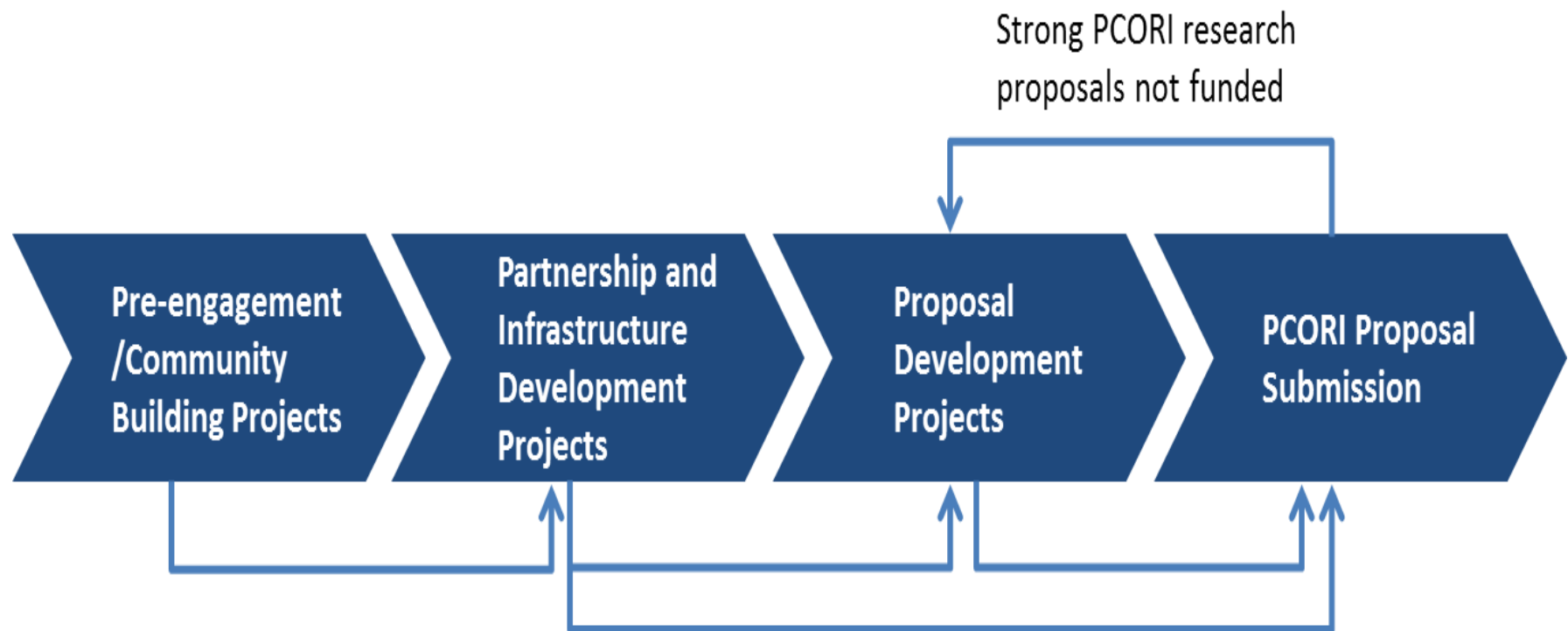
Concept Origin

- Proposed by participants at October 2012 *Transforming Patient-Centered Research* patient engagement workshop
- Workshop participants identified that few resources have been directed to non-research entities for community development, capacity building, or for infrastructure development for engagement in research as partners.

Purpose

- Build community
- Form or strengthen reciprocal relationships between researchers and non-research communities
- Support capacity building, co- learning, and the development of a sustainable infrastructure to facilitate “research done differently”
- Accelerate proposal submission (or re-submission)

Design



Pre-Engagement/Community-Building Projects (Up to \$15,000 for 6 months)

Available to individuals, consumer/patient organizations, clinician(s) or researcher(s), or a combination of the above to support:

- Community building
- Creation of structure and communication strategies
- Developing an understanding of PCORI and “research done differently”



Partnership and Infrastructure Development Projects (Up to \$30,000 for 1 year)

- Available to emerging research/non-research partnerships to support:
 - Data network development
 - Development of infrastructure
 - Generation of research questions through community events, town hall meetings, and so forth.
 - Minimum** 50% of funds go to non-research partner(s)



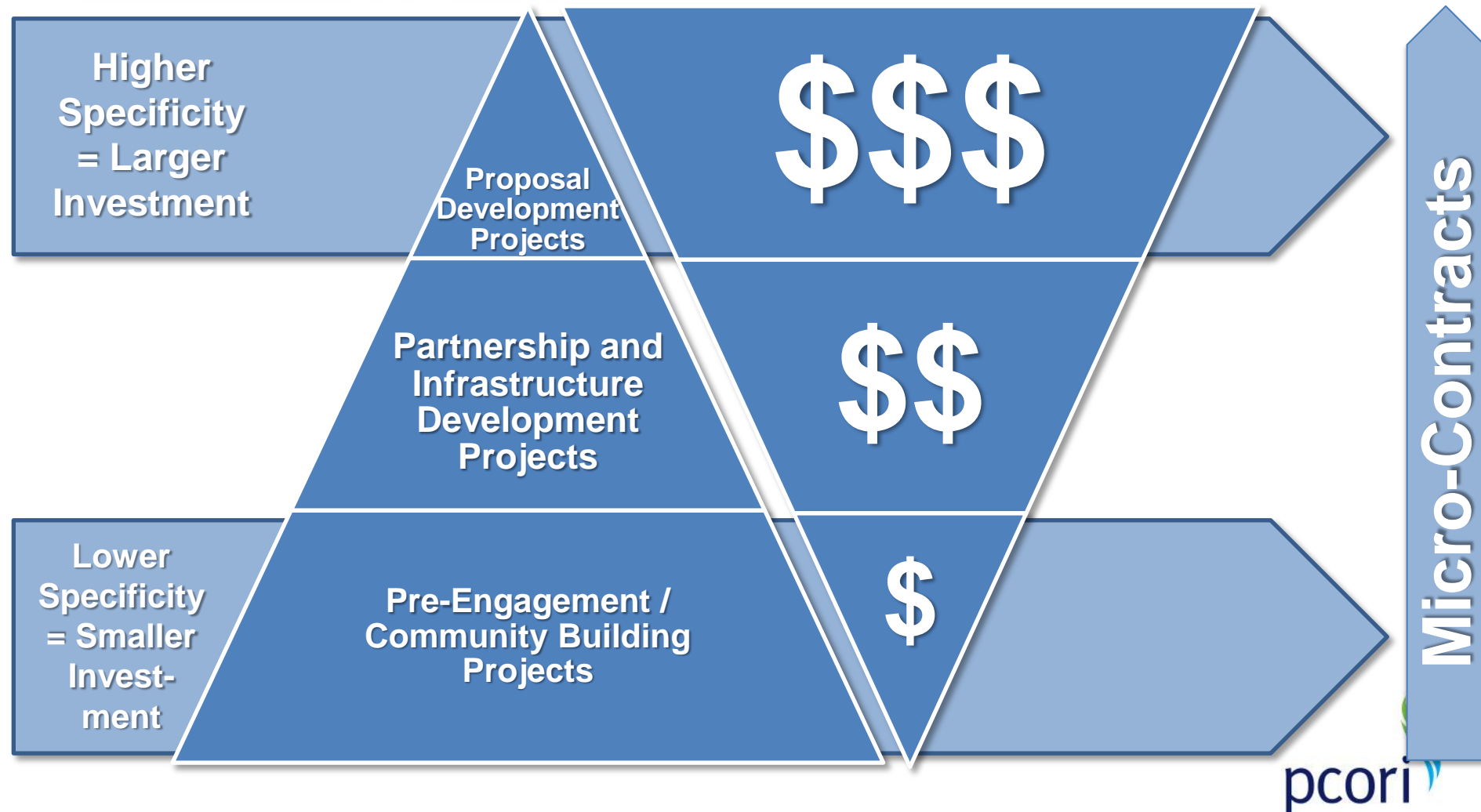
Proposal Development Projects (Up to \$50,000 for 1 year)



Available to advanced research/non-research partnerships – including those that submitted PCORI proposals and were not funded – to support:

- PCORI research proposal (re)submission
- Research partnership skill development
- **Minimum** 50% of funds go to non-research partner(s)

Relationship of funding dollars to different levels of partnerships/projects



Next Steps

- For 2013, focusing on Pre-Engagement/Community-Building Projects
- Decide on funding and administrative mechanism for these awards
- Finalize plan and launch



Discussion of the PCORI Ambassadors Program

Aingyea Kellom, MPA

Program Associate, Patient Engagement, PCORI

Advisory Panel Kickoff & Training

April 20, 2013

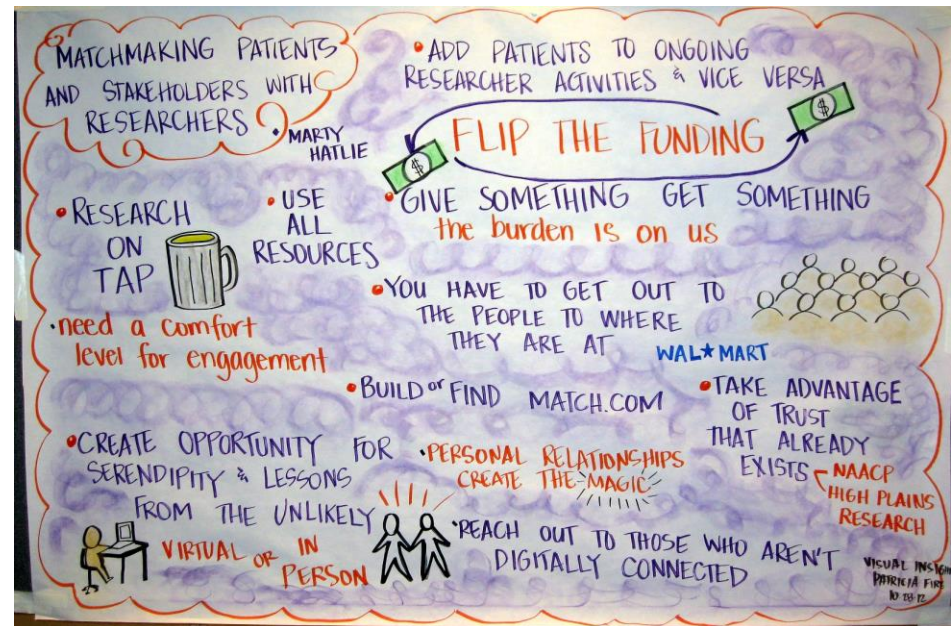
Patient-Centered Outcomes Research Institute

The Opportunity



Proposed by participants at October 2012 *Transforming Patient-Centered Research: Building Partnerships and Promising Models* workshop.

- To help PCORI reach beyond those who self-identify as patients
- To raise awareness and recruit patients, consumers, community members, and other stakeholders



The Program Purpose

- Develop a grassroots group of volunteers who are interested in educating their community about PCORI and the important role patient-centered research plays in helping individuals make informed healthcare decisions
- Within their community of influence, each ambassador helps develop trust with other individuals and groups who may be interested.
- To provide a source of knowledge and experience for PCORI
- Targeted Audience: Patients and Caregivers

How Will it Work?

- Ambassadors review the current PCORI 101 training and agree to the Ambassador pledge
- Ambassadors share PCORI information (tool kits) with their community of influence and invite them to get involved
- Ambassadors interact in an established social media outlet to connect with PCORI and other Ambassadors, develop relationships, and swap engagement stories

How Will it Work....Later?

- PCORI Ambassadors volunteer to participate in additional training to become a “Lead PCORI Ambassador” and agree to an extended Ambassador pledge
- PCORI Ambassadors continue at current activity level, Lead PCORI Ambassadors become active in increased levels such as local health fairs
- All PCORI Ambassadors interact in an established social media outlet

PCORI Ambassador Requirements



Tier 1 – PCORI Ambassador

- PCORI 101 training
- Ambassador Pledge
- Establish a presence on social media outlet
- Recruit additional PCORI Ambassadors or individuals interested in other PCORI Initiatives



Tier 2 – Lead PCORI Ambassador

- Tier 1 Requirements
- Additional Training
 - Polishing of Story
 - Media
 - Applying for PCORI Funding, such as Engagement Awards
- Contribute to Quarterly Newsletter

What's In It for You?

- Receive PCORI Ambassador training and learn about other PCORI initiatives such as PFA working groups, roundtables, and advisory panels
- Build relationships with other PCORI Ambassadors, PCORI staff, and like-minded community members
- The opportunity to co-author publications, submit guest blogs, and be highlighted in the quarterly Ambassador newsletter
- Visit organizations and events to promote the importance of PCOR, from a patient or other stakeholder perspective

Let's discuss...

- Tiered program...designed to accommodate different individuals' time constraints and interest
- Resources...training, webinars, tool kits, online community, PCORI staff
- Ambassador Profiles...engagement interest, healthcare issue, geographic area, and so forth.
- Face-to-Face Meeting...annual meeting
- What are we missing?



Defining Patient-Centeredness in Research

Suzanne Schrandt, JD

Deputy Director, Engagement, PCORI

Lori Frank, PhD

Director, Engagement Research, PCORI

Patient-Centered Outcomes Research Institute

Background

- Beginning in March, 2011, PCORI's Methodology Committee began an extensive, iterative, and transparent process to define patient-centered outcomes research including;
 - Exhaustive review of available literature
 - Focus groups including diverse representation of patients and stakeholders
 - Significant public comment period with input from 120 organizations and 450 individuals
- On March 25, 2012, the PCORI Board of Governors approved a working definition of “patient-centered outcomes research”

Patient-Centered Outcomes Research

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

“Given my personal characteristics, conditions and preferences, what should I expect will happen to me?”

“What are my options and what are the potential benefits and harms of those options?”

“What can I do to improve the outcomes that are most important to me?”

“How can clinicians and the care delivery systems they work in help me make the best decisions about my health and healthcare?”

PCOR is research that:

- Assesses the benefits and harms of preventive, diagnostic, therapeutic, palliative, 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 burdens to individuals, availability of services, technology, and personnel, and other stakeholder perspectives.



Conclusions and Next Steps

Sue Sheridan, MBA, MIM

Director, Patient Engagement, PCORI

Advisory Panel Kickoff & Training

April 20, 2013

Patient-Centered Outcomes Research Institute

Next Steps

- Sub-committees
- Chairperson selection
- Future meetings and communications