PCORI:
Studying What Works for Whom

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pcori.
PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

Who We Are
and What We Do

PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

About Us

- An independent research institute authorized by Congress in 2010 and governed by a 21-member Board of Governors representing the entire healthcare community
- Funds comparative clinical effectiveness research (CER) that engages patients and other stakeholders throughout the research process
- Seeks answers to real-world questions about what works best for patients based on their circumstances and concerns

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Why Is Our Work Needed?

- For all the advances it produces, traditional healthcare research has not answered many questions patients face.
- People want to know which preventive, diagnostic, or treatment option is best for them.
- Patients and their clinicians need information they can understand and use.

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How is Our Work Different?

- We fund research on which care options work, for whom, under which circumstances.
- We focus on answering questions most important to patients and those who care for them.
- We aim to produce evidence that can be easily applied in real-world settings.
- We engage patients, caregivers, clinicians, insurers, employers and other stakeholders throughout the research process.
- This makes it more likely we'll get the research questions right and that the study results will be useful and taken up in practice.

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Our Broad and Complex Mandate

"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...**
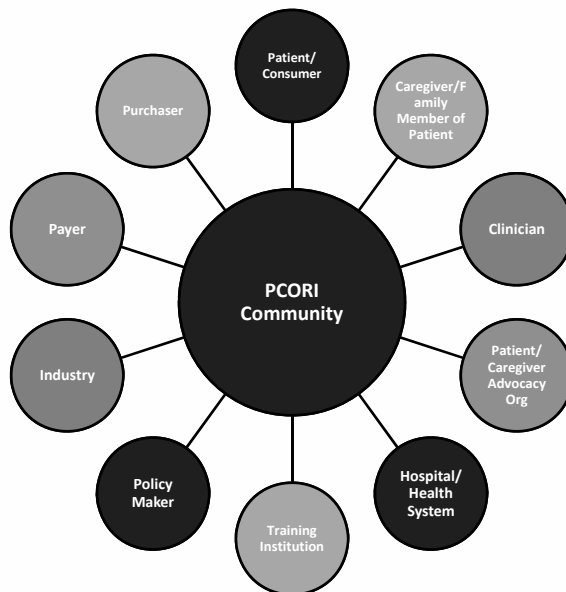
... and the dissemination of research findings with respect to the relative health outcomes, clinical effectiveness, and appropriateness of the medical treatments, services..."

--from PCORI's authorizing legislation

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Who Are Our Stakeholders?

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Our Research Focus

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We Fund CER

Research that….

- Generates and synthesizes evidence comparing benefits and harms of at least two different methods to prevent, diagnose, treat, and monitor a clinical condition or improve care delivery
- Measures benefits in real-world populations
- Informs a specific clinical or policy decision
- Describes results in subgroups of people
- Applies appropriate methods and data sources
- Helps consumers, clinicians, purchasers, and policy makers make informed decisions that will improve care for individuals and populations

Adapted from *Initial National Priorities for Comparative Effectiveness Research*, Institute of Medicine of the National Academies

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We Fund Patient-Centered Outcomes Research

PCOR is a relatively new form of CER that….

- Considers patients' needs and preferences, and the outcomes most important to them
- Investigates what works, for whom, under what circumstances
- Helps patients and other healthcare stakeholders make better-informed decisions about health and healthcare options

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Our National Priorities for Research

Assessment of Prevention, Diagnosis, and Treatment Options

Research that:

- Compares the effectiveness and safety of alternative prevention, diagnosis, and treatment options
- Determines which ones work best for different people with a particular health problem

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Our National Priorities for Research

Assessment of Prevention, Diagnosis, and Treatment Options

Improving Healthcare Systems

Research that:

- Compares health system–level approaches to improving access
- Supports patient self-care, innovative use of health information technology, care coordination for complex conditions, and effective workforce deployment

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Our National Priorities for Research

Assessment of Prevention, Diagnosis, and Treatment Options

Improving Healthcare Systems

Communication and Dissemination Research

Research on:

- Providing information produced by CER
- Empowering people to ask for and use the information
- Supporting shared decision making between patients and their providers

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Our National Priorities for Research

Assessment of Prevention, Diagnosis, and Treatment Options

Improving Healthcare Systems

Communication and Dissemination Research

Addressing Disparities

Research on:

- Prevention, diagnosis, or treatment effectiveness
- Preferred clinical outcomes across patient populations
- Health care required to achieve best outcomes in each population

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Our National Priorities for Research

Assessment of Prevention, Diagnosis, and Treatment Options

Improving Healthcare Systems

Communication and Dissemination Research

Addressing Disparities

Accelerating Patient-Centered Outcomes Research and Methodological Research

Research on:

- Building data infrastructure
- Improving analytic methods
- Training researchers, patients, and other stakeholders to participate in this research

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We Fund Research That…

Meets these criteria:

1: Impact of the condition on health of individuals and populations
Is associated with a *significant burden in the US population* (prevalence, mortality, morbidity, individual suffering, or loss of productivity)

2: Potential for the study to improve health care and outcomes
Has the potential to lead to *meaningful improvement in the quality and efficiency of care* and to improvements in outcomes important to patients

3: Technical merit
Has a research design of sufficient technical merit to ensure that the *study goals will be met*

4: Patient-centeredness
Focuses on questions relevant to *outcomes of interest to patients and caregivers*

5: Patient and stakeholder engagement
Includes patients and other healthcare stakeholders as *partners in every stage of the research*

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We Fund Research That…

What we mean by…

"Patient-centeredness"

- The project aims to answer questions or examine outcomes that matter to patients within the context of patient preferences
- Research questions and outcomes should reflect what is important to patients and caregivers

"Patient and stakeholder engagement"

- Patients are partners in research, not just "subjects"
- Active and meaningful engagement between scientists, patients, and other stakeholders
- Community, patient, and caregiver involvement already in existence or a well-thought-out plan

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We Fund Research That…

Targets specific, high-priority topics, including:

- PCOR treatment options in uterine fibroids*
- Multifactorial fall injury prevention strategy in older persons**
- Effectiveness of transitional care
- Treatment options for African Americans and Hispanics/Latinos with uncontrolled asthma
- Obesity treatment options set in primary care for underserved populations
- Clinical interventions to address hypertension disparities**

*Project administered by AHRQ
**Project administered by NIH

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We Also Work to Improve Research Methodology

In any study, methods matter. That's why we've developed methodology standards that patient-centered CER should follow, at a minimum.

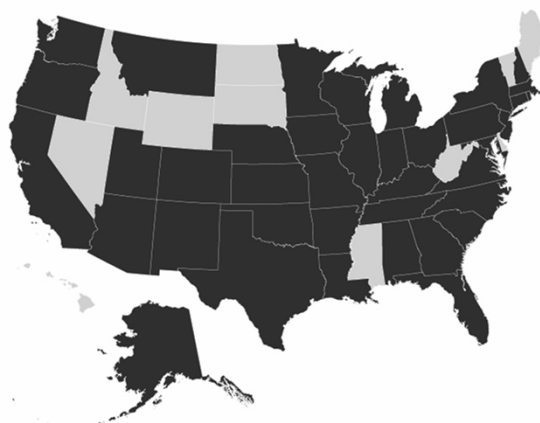**Methodology Standards: 11 Broad Categories**

- Formulating Research Questions
- Patient-Centeredness
- Data Integrity and Rigorous Analyses
- Preventing/Handling Missing Data
- Heterogeneity of Treatment Effects

- Data Networks
- Data Registries
- Adaptive and Bayesian Trial Designs
- Causal Inference
- Studies of Diagnostic Tests
- Systematic Reviews

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Support for Researchers Nationwide

Number of projects: 399

Amount awarded: $854.8 million

Number of states where we are funding research:
39 (plus the District of Columbia)

As of April 21, 2015

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Our Growing Research Portfolio

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Portfolio Analysis: Primary Condition

Where are funded projects concentrated?

38	Cancer
38	Mental/ Behavioral Health
22	Cardiovascular Health
18	Neurological Disorders
17	Respiratory Diseases
17	Nutritional and Metabolic Disorders
17	Rare Disease

Where is the most funding (in millions)?

$108.9	Cancer
$70.6	Trauma/Injury
$68.4	Mental/ Behavioral Health
$50.9	Cardiovascular Health
$48.7	Nutritional and Metabolic Disorders
$44.1	Rare Disease
$43.2	Respiratory Diseases

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A Comparison of Nonsurgical Treatment Methods for Patients with Lumbar Spinal Stenosis

Engagement

- Questionnaires before and after treatment will collect patient-reported outcomes

Potential Impact

- Could change practice by determining whether there are any examination findings that could be used to predict which type of patient would do better with which type of treatment for lumbar spinal stenosis

Methods

- Randomized controlled trial

Compares the effectiveness of three common nonsurgical treatment approaches for stenosis:
1) usual medical care that involves prescription drugs and/or injections into the spine;
2) group exercise in supervised community center; and 3) hands-on (manual) therapy and rehabilitative exercises in a clinical setting.

Principal Investigator:
Michael J. Schneider, DC, PhD,
University of Pittsburgh
Pittsburgh, PA

Assessment of Prevention, Diagnosis, and Treatment Options,
awarded December 2012

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Pragmatic Clinical Studies

Seeks to produce information that can be directly adopted by providers:

- Compares two of more options for prevention, diagnosis, treatment, or management of a disease or symptom
- Addresses critical clinical choices faced by patients, caregivers, clinicians, and systems
- Often conducted in routine clinical settings
- Though often large, usually less complex protocols than traditional trials
- Topics of special interest from stakeholders, Institute of Medicine, Agency for Healthcare Research and Quality

Overview

- Anticipated Awards per Funding Cycle: Six to Nine
- Funds Available per Cycle: Up to $90 Million
- Maximum Project Duration: 5 Years
- Maximum Direct Costs per Project: $10 Million

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Pragmatic Clinical Study Awards

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Targeted Interventions to Prevent Chronic Low Back Pain in High-Risk Patients: A Multi-Site Pragmatic RCT

Engagement

- Engagement will involve payers, EMR system vendors, national primary care and physical therapy (PT) professional societies, and delivery system organizations, including PCP practices and PT clinician groups and the American Chronic Pain Association

Potential Impact

- Could change and improve clinical practices for lower back pain

Methods

- Multi-site cluster randomized pragmatic clinical trial, with 60 clinical practices across 5 regional areas

Determines whether a prompt referral to evidence-based PT, augmented with cognitive behavioral principles, for high-risk patients presenting with acute lower back pain is more effective in preventing the progression to chronic lower back pain.

Principal Investigator:
Anthony Delitto, PhD, PT
University of Pittsburgh
Pittsburgh, PA

Large Pragmatic Studies to Improve
Patient-Centered Outcomes, awarded February 2015

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Early Supported Discharge for Improving Functional Outcomes after Stroke

Engagement

- Patients, caregivers, and other stakeholders guided project planning and will be engaged continually at both state and community levels

Potential Impact

- Could fill an evidence gap for an effective post-acute care model that specifically addresses the needs of stroke patients and their caregivers across the country

Methods

- Pragmatic cluster RCT in 50 North Carolina (NC) hospitals

Building on the successful NC Stroke Care Collaborative (NCSCC) registry, a prospective stroke database in which 51 (of 113) hospitals in NC enroll patients, this pragmatic cluster RCT of 50 NC hospitals aims to determine the effectiveness of comprehensive post-acute stroke services (COMPASS), a patient-centered intervention uniting transitional care management services and elements of early supported discharge in stroke patients discharged directly home.

Principal Investigator:
Pamela Duncan, PhD, PT
Wake Forest University
Winston-Salem, NC

Large Pragmatic Studies to Improve Patient-Centered Outcomes, awarded February 2015

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The National Patient-Centered Clinical Research Network (PCORnet)

11 Clinical Data Research Networks

Health system-based networks, such as hospital systems; $76.8 million awarded

18 Patient-Powered Research Networks

Patients with a single condition form a research network; $16.8 million awarded

Coordinating Center

Provides technical and logistical assistance under the direction of a steering committee and PCORI staff

pcornet
The National Patient-Centered Clinical Research Network

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The National Patient-Centered Clinical Research Network (PCORnet)

- Improve the nation's capacity to conduct clinical research faster, more efficiently and less expensively, with greater power
- Establish a large, highly representative, national patient-centered clinical research network with a focus on conducting randomized and observational comparative studies
- Support a learning US healthcare system, which would allow for large-scale research to be conducted with greater accuracy and efficiency within real-world care-delivery systems
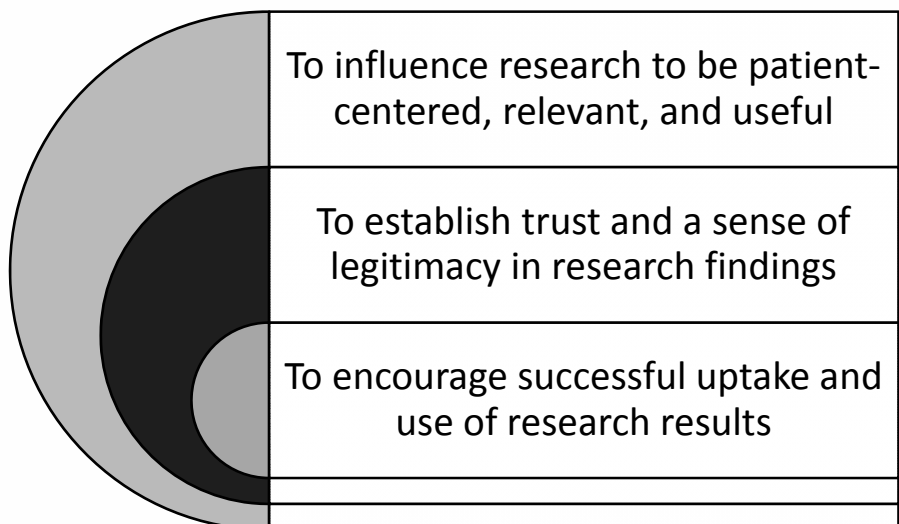
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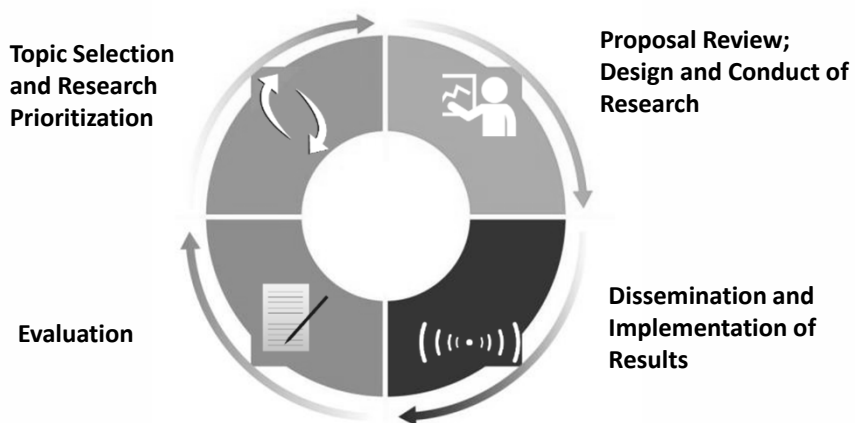

Better Research Through Engagement

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Why Engage?

	To influence research to be patient-centered, relevant, and useful
	To establish trust and a sense of legitimacy in research findings
	To encourage successful uptake and use of research results

Engagement as a Path to Useful, High-Quality Research

Topic Selection and Research Prioritization

Proposal Review; Design and Conduct of Research

Evaluation

Dissemination and Implementation of Results

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Our Engagement Rubric–A Valuable Resource

Provides practical guidance to applicants, merit reviewers, awardees, and engagement/program officers on effective engagement in research

- **Planning the Study:** How patient and stakeholder partners will participate in study planning and design

- **Conducting the Study:** How patient and stakeholder partners will participate in the conduct of the study

- **Disseminating the Study Results:** How patient and stakeholder partners will be involved in plans to disseminate study findings and ensure that findings are communicated in understandable, usable ways

- **PCOR Engagement Principles:** Reciprocal relationships, co-learning, partnership, trust, transparency, honesty
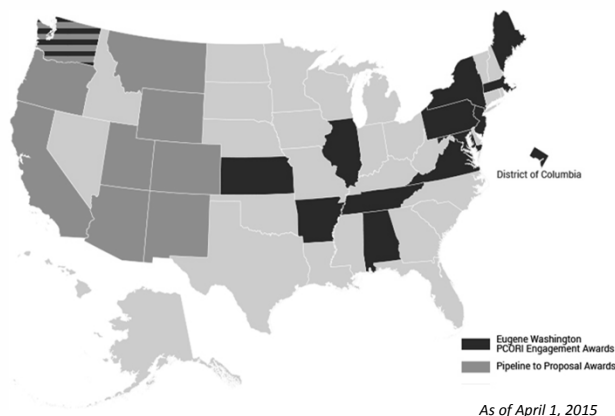
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Building a PCOR Community Nationwide

Our *Eugene Washington PCORI Engagement Awards* and *Pipeline to Proposal Awards* are not research awards but opportunities to build PCOR capacity.

Number of awards:
82

Amount awarded:
$5.8 million

Number of states where we are funding projects:
22 (and the District of Columbia)

Eugene Washington PCORI Engagement Awards

Pipeline to Proposal Awards

As of April 1, 2015

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Eugene Washington PCORI Engagement Awards

Support projects that:

- Increase knowledge about how consumers of healthcare information view, receive, and make use of PCOR/CER **(Knowledge Awards)**

- Build capacity for participating in PCOR/CER and create ways to connect patients, caregivers, and other stakeholders with the research community **(Training and Development Awards)**

- Develop and strengthen channels for disseminating and implementing PCOR and CER findings **(Dissemination Awards)**

- Provide meeting and conference support **(Engagement Award Initiative Notices)**

Overview

- 25 Projects
- $4.7 Million Awarded

As of April 1, 2015

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Pipeline to Proposal Awards

Support projects that:

- Strengthen relationships between researchers, patients, and stakeholders in communities underrepresented in research

- Build capacity for community partnerships to ultimately submit research questions to considered for PCORI funding

- Engage partnerships in the research process and development of dissemination and implementation plans

- Establish an infrastructure for dissemination and evaluation of CER information

Overview

- 57 Awards
- $1.1 Million Awarded

As of April 1, 2015

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Thank You

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