Research Done Differently: The Ins and Outs of Patient-Centered Outcomes Research

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Clinical Directors Network, Inc.

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PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

Roadmap

- Inside PCORI

- Focus on the Addressing Disparities Research Priority Area

- Research Funding Opportunities at PCORI

- How We Review Research Proposals

- Engagement Funding Opportunities at PCORI

- Q&A

Inside PCORI

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

Our Mission and Strategic Goals

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PCORI helps people make informed healthcare decisions, and improves healthcare delivery and outcomes, by producing and promoting high-integrity, evidence-based information that comes from **research guided by patients, caregivers, and the broader healthcare community.**

Our Strategic Goals:

- Increase quantity, quality, and timeliness of useful, trustworthy research information available to support health decisions

- Speed the implementation and use of patient-centered outcomes research evidence

- Influence research funded by others to be more patient-centered

PCORI's Work Answers Patients' Questions

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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?"

# Key Components of the Research We Fund

Comparative Effectiveness Research

- Directly **compares two or more** commonly used **options** to screen, prevent, diagnose, or treat conditions

- Looks at whether **A or B is better** and **for which patients**

- **Fills evidence gaps** that are important to patients and other key stakeholders

- Is conducted in **real-world populations** and **real-world settings**
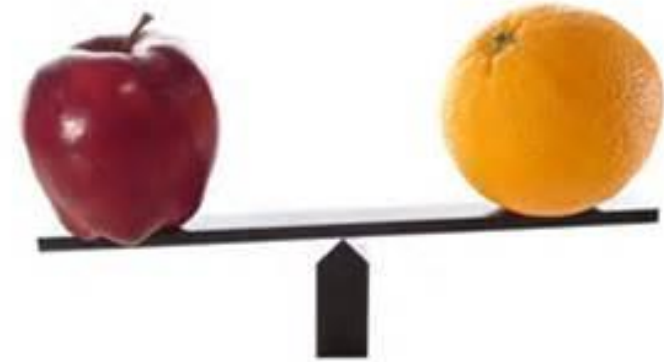
Patient-Centeredness

- Aims to answer questions or examine outcomes that matter to patients within the context of patient preferences and interests

- Research questions and outcomes reflect what is important to patients and caregivers
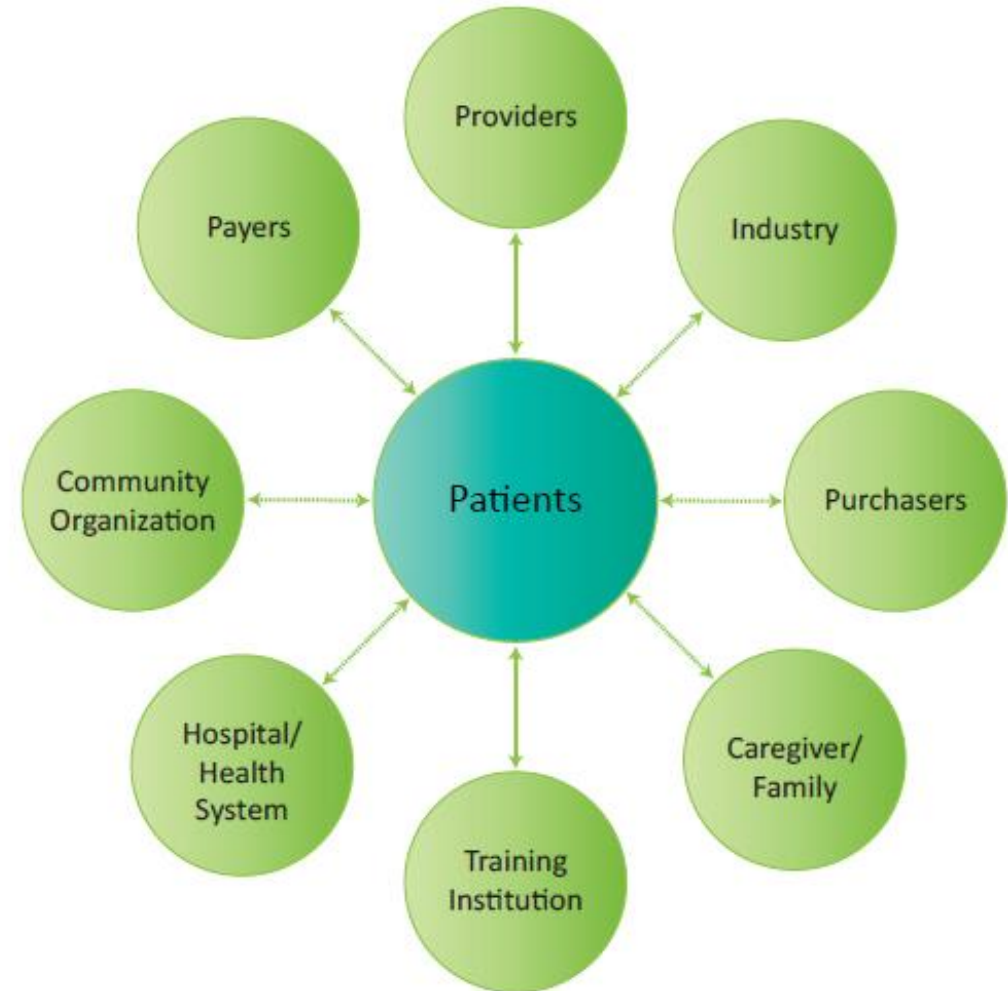
Patient and Stakeholder Engagement

- Engages scientists, patients, and other stakeholders in an active and meaningful way throughout the entire study

- Influences research to be patient-centered, relevant, and useful

- Encourages successful uptake and use of research results

Change in Study Interventions

Refocus Outcomes

Boost Recruitment

Share Their Story/Present Study Findings

Our National Priorities for Research

Assessment of Prevention, Diagnosis, and Treatment Options

Improving Healthcare Systems

Communication & Dissemination Research

Addressing Disparities
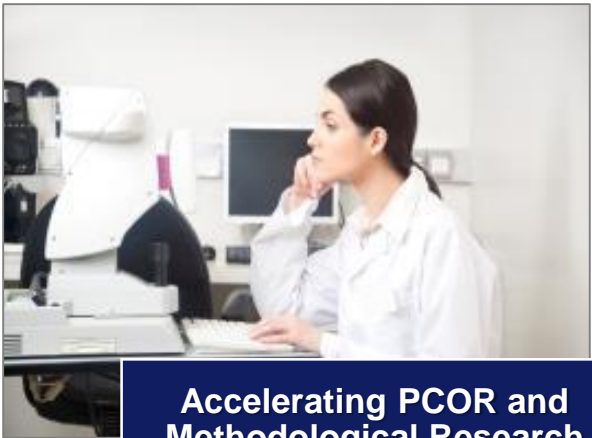
Accelerating PCOR and Methodological Research

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

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

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 at risk for disparities

- Health care required to achieve best outcomes in each population

Our National Priorities for Research

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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

Snapshot of Funded Projects
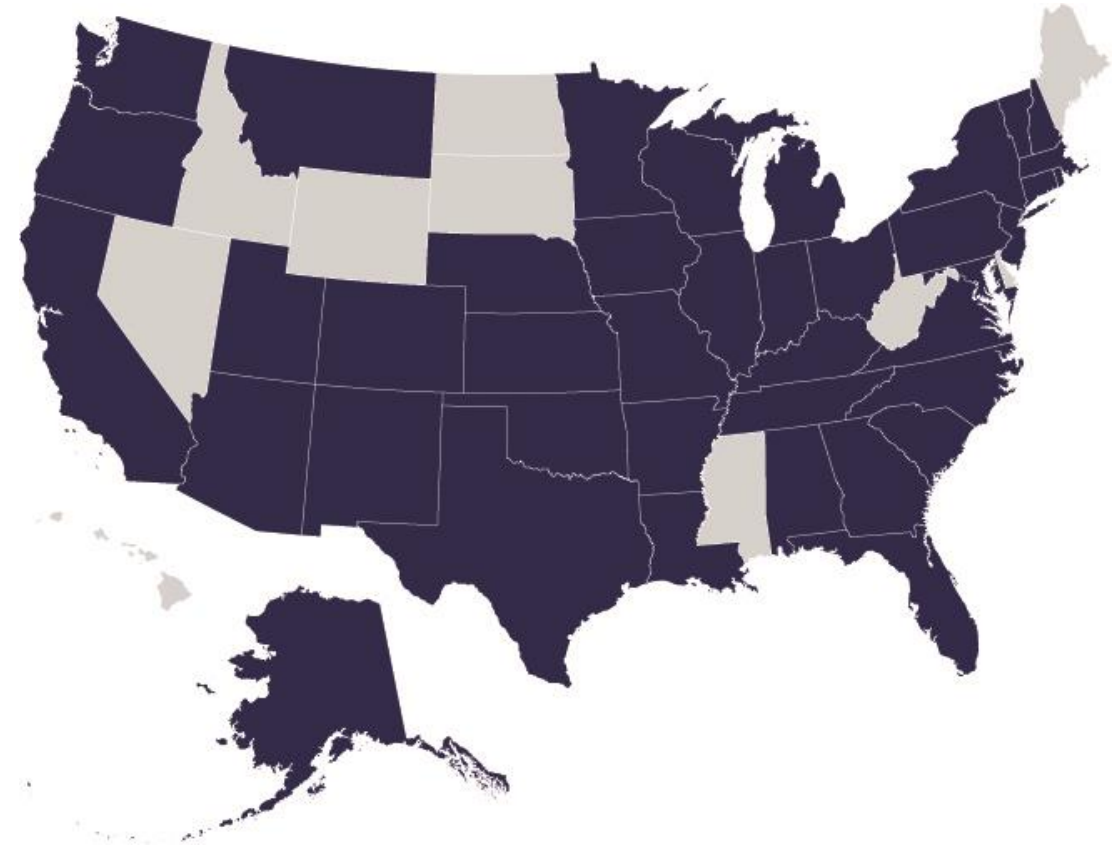
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Number of funded awards:

More than 600

Amount awarded:

More than $2 billion
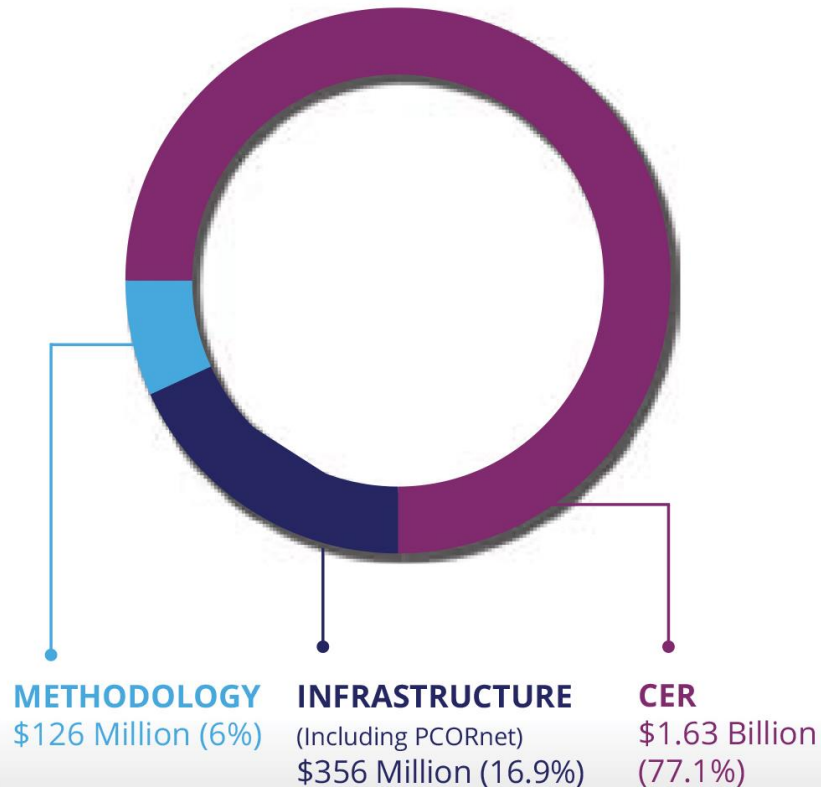
Number of states where we are funding projects:
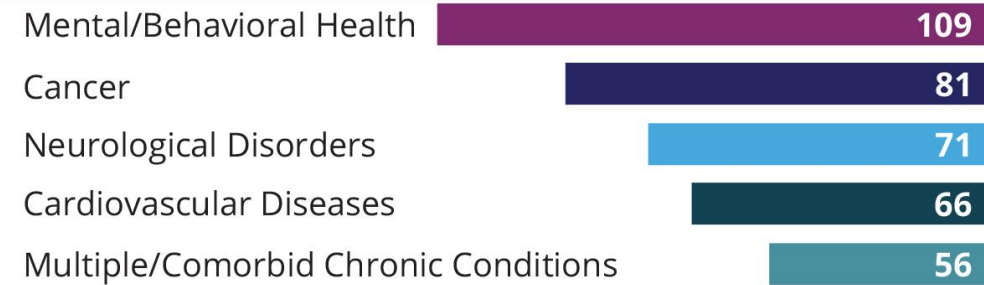
41 (plus the District of Columbia)

About Our Research Portfolio

BY THE NUMBERS

Research Projects By Area

METHODOLOGY
$126 Million (6%)

INFRASTRUCTURE
(Including PCORnet)
$356 Million (16.9%)

CER
$1.63 Billion
(77.1%)

Most Studied Conditions*

Condition	Count
Mental/Behavioral Health	109
Cancer	81
Neurological Disorders	71
Cardiovascular Diseases	66
Multiple/Comorbid Chronic Conditions	56

Most Studied Populations of Interest*

Population	Count
Racial/Ethnic Minorities	280
Low Socioeconomic Status	186
Older Adults	137
Women	134
Individuals with Multiple Chronic Conditions	105
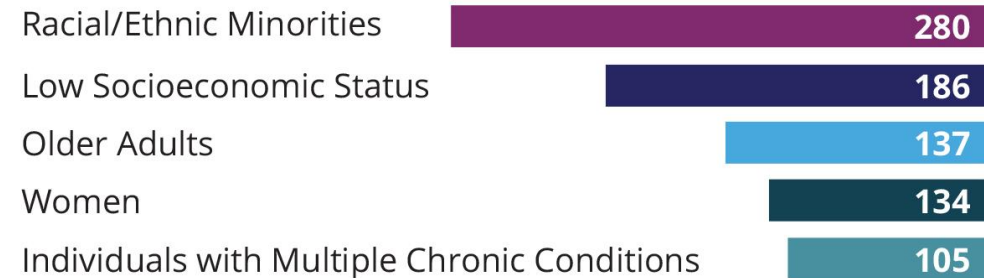
*Number of projects (out of a total of 431). A project may study more than one condition or population of interest.

PCORI Funds Research on High-Cost, High-Impact Health Conditions

National Per Capita Expenditure

Condition	Number of PCORI Studies	PCORI Investment
Stroke	10	$41 M
Heart Failure	14	$68 M
Hepatitis (Chronic Viral B and C)	8	$59 M
Chronic Obstructive Pulmonary Disease	17	$89 M
Schizophrenia/Other Psychotic Disorders	11	$22 M
Chronic Kidney Disease	16	$67 M
Asthma	16	$51 M
Atrial Fibrillation	3	$5 M
Alzheimer's Disease/Dementia	12	$64 M
Depression	35	$122 M
Cancer	77	$285 M
Ischemic Heart Disease	19	$94 M
Osteoporosis	1	$14 M
HIV/AIDS	4	$8 M
Arthritis	13	$43 M
Diabetes	29	$68 M

$32,027

$15,920

PCORI has funded **224** studies determining what works best in care for the 16 highest cost conditions in the United States.

High-cost clinical conditions identified by the Centers for Medicare and Medicaid Services for 2015.

The PCORI CER portfolio includes 392 active/completed studies funded as of September 12, 2017.

Analysis excludes methods studies, Pilots, PPRNs, CDRNs, and Engagement Awards.

Focus on the Addressing Disparities Research Priority Area

Addressing Disparities Research Priority Area Mission

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PCORI's Vision, Mission, Strategic Plan

Addressing Disparities Priority Area Mission Statement

To reduce disparities in healthcare outcomes and advance equity in health and healthcare

Guiding Principle

To support comparative effectiveness research that will identify best options for reducing and eliminating disparities

Addressing Disparities Portfolio Goals

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Identify Research Questions

- **Identify** high-priority **research questions** relevant to reducing and eliminating disparities in health and healthcare outcomes

Fund Research

- **Fund** comparative effectiveness **research** with the highest potential to reduce and eliminate health and healthcare disparities

Disseminate Promising/Best Practices

- **Disseminate** and facilitate the adoption of **promising/best practices** to reduce and eliminate health and healthcare disparities

Snapshot of the Addressing Disparities Portfolio
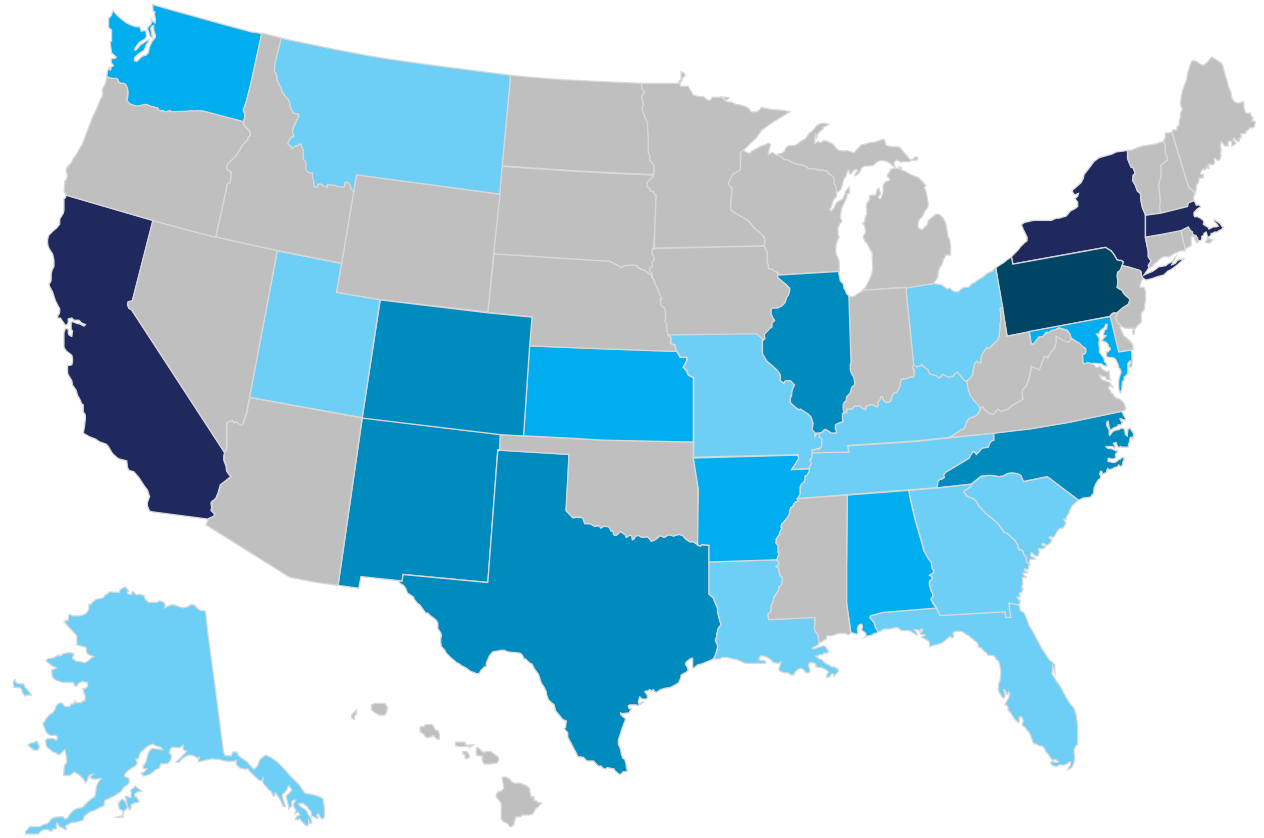
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Number of funded awards:
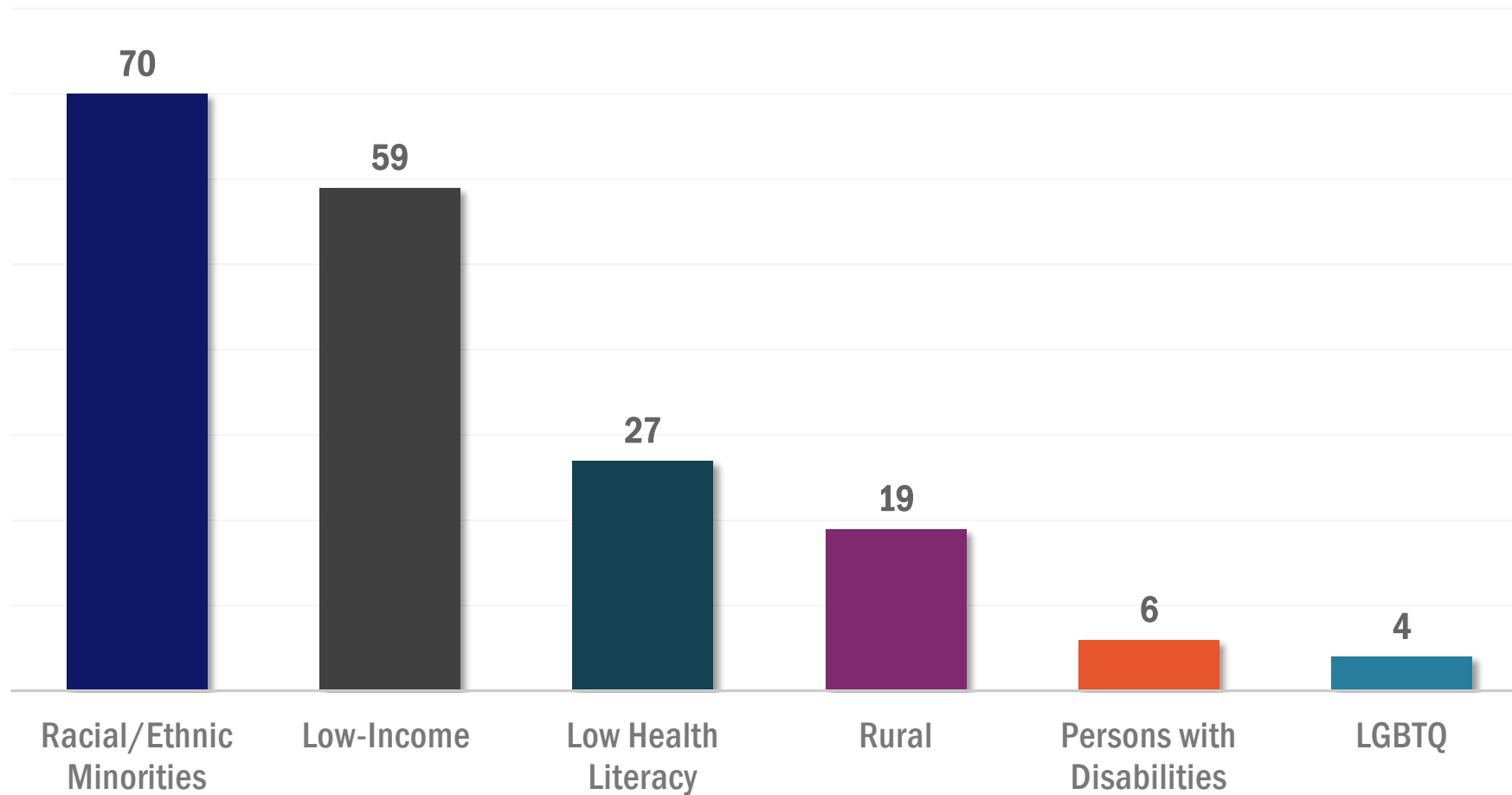
81

Amount awarded:

$250 million

Number of states where we are funding projects:

25 (plus the District of Columbia)

Addressing Disparities Populations of Interest

of Projects in Portfolio

Population	# of Projects
Racial/Ethnic Minorities	70
Low-Income	59
Low Health Literacy	27
Rural	19
Persons with Disabilities	6
LGBTQ	4

Addressing Disparities Portfolio by Condition

Condition	# of Projects
Mental/Behavioral Health	16
Respiratory Diseases	11
Nutritional and Metabolic Disorders	10
Cardiovascular Health	8
Neurological Disorders	6
Multiple/co-morbid chronic conditions	5
Cancer	4
Reproductive and Perinatal Health	2
Liver Disease	2
Functional Limitation and Disabilities	1
Other	16
Total	81

Long-Term Outcomes of Community Engagement to Address Depression Outcomes Disparities

Engagement

- Community agencies collaborate to tailor depression toolkits to needs and strengths of community.

Methods

- Both a mixed-methods approach and a randomized controlled trial

Results

- The two approaches didn't differ in people's depression or mental health quality of life. People in programs assigned to the coalition approach showed more improvement in physical health quality of life and averaged fewer nights in the hospital for mental health problems.

Project looks at whether a community engagement intervention or technical assistance model will improve clients' mental health and physical functioning and reduce risk factors for homelessness. It will also identify patient preferences and priorities and assess community capacity to respond to these priorities.

Kenneth Brooks Wells, MD, MPH University of California, Los Angeles

Addressing Disparities, awarded December 2012

Research Funding Opportunities at PCORI

PCORI Funding Opportunities

Our research funding is awarded through PCORI Funding Announcements.

Open opportunities are posted at **pcori.org/apply**.

PCORI Comparative Effectiveness Research Funding Opportunities

1 Broad Funding Announcements

2 Targeted Funding Announcements

http://www.pcori.org/funding-opportunities

Broad Funding Announcement

- Supports research on investigator initiated research topics that address questions of importance to patients and other stakeholders.

- The announcement includes 5 different types of awards that align with PCORI's National Priorities for Research

- Awards range from $750,000 – $5,000,000 in direct costs per project and are generally 3 years in duration

- Upcoming Deadlines
  - Letter of Intent: **November 6, 2018**; Application: **January 16,2019**
  - Letter of Intent: **January 31, 2019**; Application: **April 24, 2019**

Targeted Funding Announcement

- Seek research proposals on the highest-priority questions identified through PCORI's topic generation and research prioritization process

- Examples:

  – Clinical Management of Hepatitis C Infection

  – Treatment of Multiple Sclerosis

  – Management of Care Transitions for Emerging Adults with Sickle Cell Disease

- Budget and project duration vary by funding announcement

PCORI CER Funding Announcements
Contacting Program Staff

- http://www.pcori.org/funding-opportunities
  - Broad PCORI Funding Announcements
  - Targeted PCORI Funding Announcements
- Contact PCORI
  - **Programmatic Inquires:**
    Email: sciencequestions@pcori.org
    Phone: 202-627-1884
    Research Inquiry Form: http://www.pcori.org/PFA/inquiry
  - **Administrative, Financial, or Technical Inquiries:**
    Email: pfa@pcori.org
    Phone: 202-627-1885

How We Review Research Proposals

Our Merit Review Process

1 **Evaluate Letters of Intent.** At least two PCORI staff review each Letter of Intent and a committee of staff members determines which will be invited to submit full applications. Information in the Letters of Intent drives the recruitment of patients, clinicians, scientists, and other members of the healthcare community who have the appropriate expertise to serve on merit review panels.

2 **Preliminary review of full applications.** Reviewers conduct a preliminary review of applications, providing written critiques and scores for the applications based on our review criteria and scoring system.

Our Merit Review Process

3 **In-person panel review.** Following the preliminary review, a subset of applications move forward and are discussed and scored by the full panel of merit reviewers at an in-person meeting. The perspectives of a wide range of reviewers help us choose projects that are important, practical, and relevant to the audiences who will use the results of our work.

4 **Selection Committee Review.** Program staff recommend projects to a Selection Committee that includes members of PCORI's Board of Governors. The committee identifies a slate of applications for possible funding based on merit review scores, programmatic balance and fit, and PCORI's strategic priorities.

This slate is proposed to PCORI's Board for consideration and approval.

Our Merit Review Process

	Patient/Other Stakeholder Reviewers	Scientist Reviewers
Criterion 1: Potential for the study to fill critical gaps and generate actionable evidence		✓
Criterion 2: Potential for the study findings to be adopted into clinical practice and improve delivery of care	✓	✓
Criterion 3: Scientific merit (research design, analysis, and outcomes)		✓
Criterion 4: Investigator(s) and environment		
Criterion 5: Patient-centeredness	✓	✓
Criterion 6: Patient and stakeholder engagement	✓	✓

Tips for Submitting LOIs and Applications

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- **Read PFAs carefully**

- **Speak/email with PCORI program staff** early and as needed

- Compare your proposal with **PCORI-funded studies** on your topic/condition/intervention to identify differences and call out the evidence gaps filled by your proposal

- Cite **systematic reviews and guidelines** that identify current gaps in the evidence and document that the topic is a high-priority for patient and other stakeholder partners, PCORI, Institute of Medicine, or the Agency for Healthcare Quality and Research

Tips for Submitting LOIs and Applications

- Describe the **potential** for your study to **improve the quality of the evidence** available

- Highlight how/why this **study is of interest** to patients and other stakeholders

- Consider appropriate and patient-centered primary and secondary outcomes, including **patient-reported and clinical outcomes**

- Identify how your **intervention components** will address the identified research gap

- Describe selected **intervention(s)** and **comparator(s)** and provide evidence of proven **efficacy, effectiveness, or wide-spread use** (e.g., cite published literature, conduct pilot study provide data, document use in clinical setting)
    - If usual care is a comparator, describe why it is appropriate and how it will be delivered constantly

Tips for Submitting LOIs and Applications

- Identify appropriate stakeholder partners that can influence the conduct of CER and the uptake and use of research findings

- Describe **engagement** of patient and stakeholder partners in **developing the research question and proposal**

- Describe how your will **engage** patients and other stakeholders **over the life cycle of your project**, from planning and conducting the study to disseminating results

- Describe **roles, responsibility, and compensation** of patient and other stakeholder partners

- Use PCORI's Engagement Rubric to help structure your engagement plan: http://www.pcori.org/sites/default/files/Engagement-Rubric.pdf

Engagement Funding Opportunities at PCORI

Building a PCOR Community Nationwide

Our Eugene Washington PCORI Engagement Awards and Pipeline to Proposal Awards are not research awards but opportunities to build patient-centered outcomes research capacity.

Number of awards:

285*

Amount awarded:

$57 million

States with funded projects:

46(plus DC and Puerto Rico)

These awards support projects that promote meaningful involvement by patients, caregivers, clinicians, and other healthcare stakeholders in comparative clinical effectiveness research (CER).

*AS OF APRIL 23, 2018

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Engagement Award Funding Opportunities

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Up to $300,000 2 years

Engagement Award: Dissemination Initiative
Objective: Actively disseminate PCORI-funded research findings
Next Letter of Intent due date: October 1, 2018

Up to $50,000 1 year

Engagement Award: Conference Support
Objective: Convene stakeholders and communicate PCORI-funded research finding to a targeted end-user audience
Next Application due date: October 1, 2018

Up to $250,000 2 years

Engagement Award: Capacity Building
Objective: Develop infrastructure and partnerships for dissemination and implementation of PCORI-funded research findings
Next Letter of Intent due date: October 1, 2018

PCORI Engagement Awards
Contacting Program Staff

- https://www.pcori.org/funding-opportunities/research-support-funding-opportunities/eugene-washington-pcori-engagement

- Contact PCORI

  - **Programmatic and Administrative Inquires:**
    Email: ea@pcori.org
    Phone: 202-370-9312

Q&A

Contact Information

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