

A photograph of two people walking on a beach at sunset. The sun is low on the horizon, creating a warm orange glow. The water is calm, reflecting the sunset. Several dark, flat rocks are scattered in the shallow water, forming a path. The two people are walking away from the camera, towards the horizon. The person on the left is wearing a dark jacket and pants, and the person on the right is wearing a dark jacket and pants. The text "Driving Toward Health Impact: Our Journey and Path Forward" is overlaid on the left side of the image in a large, white, sans-serif font.

# Driving Toward Health Impact: Our Journey and Path Forward

NIH Collaboratory Grand Rounds  
October 2, 2020

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Executive Director  
Patient-Centered Outcomes  
Research Institute

# Our Discussion Today

- PCORI mission and portfolio
- Evolving healthcare landscape
- Health disparities and COVID-19
- PCORI 2.0
- Comparative clinical effectiveness trials



# Our Mission



- Authorized by Congress in 2010, governed by healthcare stakeholders
- Funds comparative clinical effectiveness research engages patients and other stakeholders
- Answers real-world questions

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 Accomplishments

## Funding and Impact

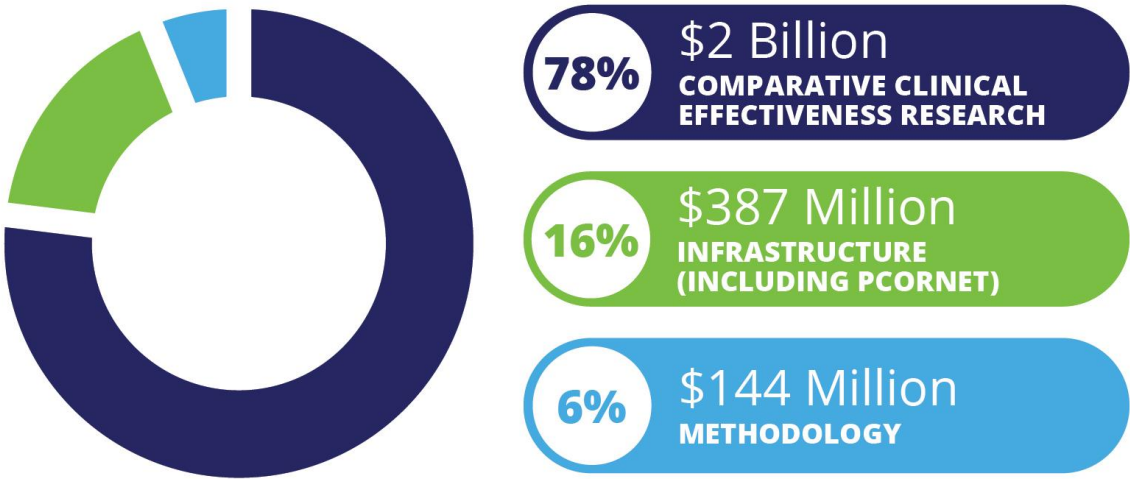


AS OF MARCH 2020, PCORI HAS COMMITTED

**\$2.6** **BILLION**  
**TO FUND** **>1,500**

RESEARCH, DISSEMINATION & IMPLEMENTATION  
and ENGAGEMENT PROJECTS

FUNDED PROJECTS BY AREA



As of March 2020



# PCORI-Funded Research Projects: Conditions and Populations of Interest

## MOST STUDIED CONDITIONS

Number of Projects: 493 Total

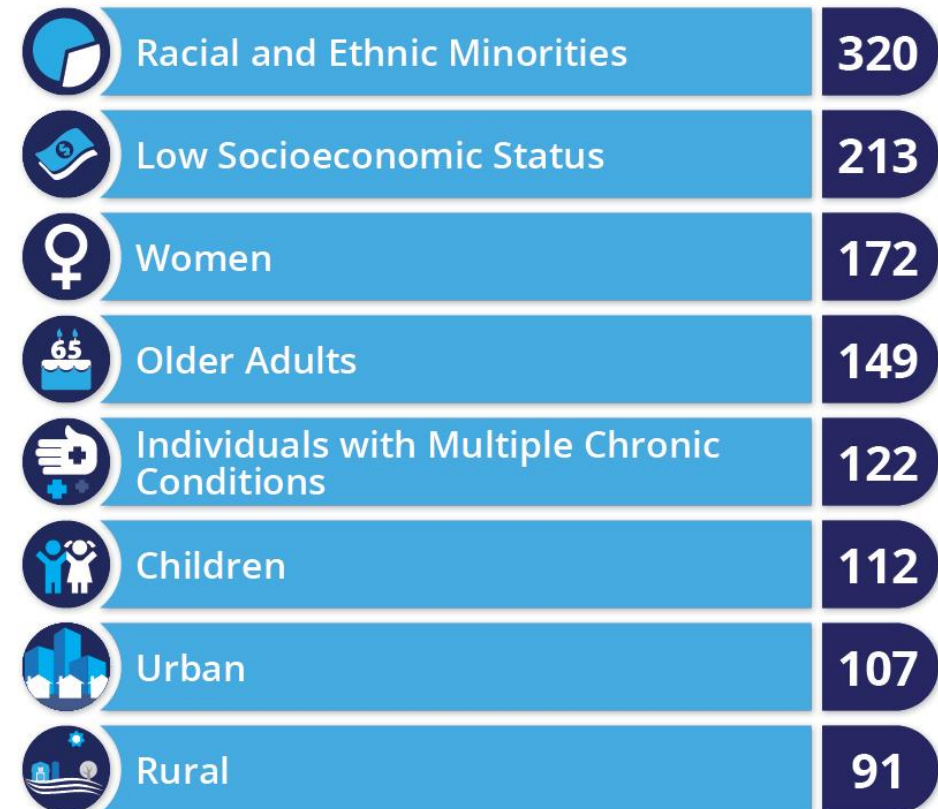


A project may address more than one condition.

As of March 2020

## POPULATIONS OF INTEREST

Number of Projects: 493 Total



A project may address more than one population of interest.

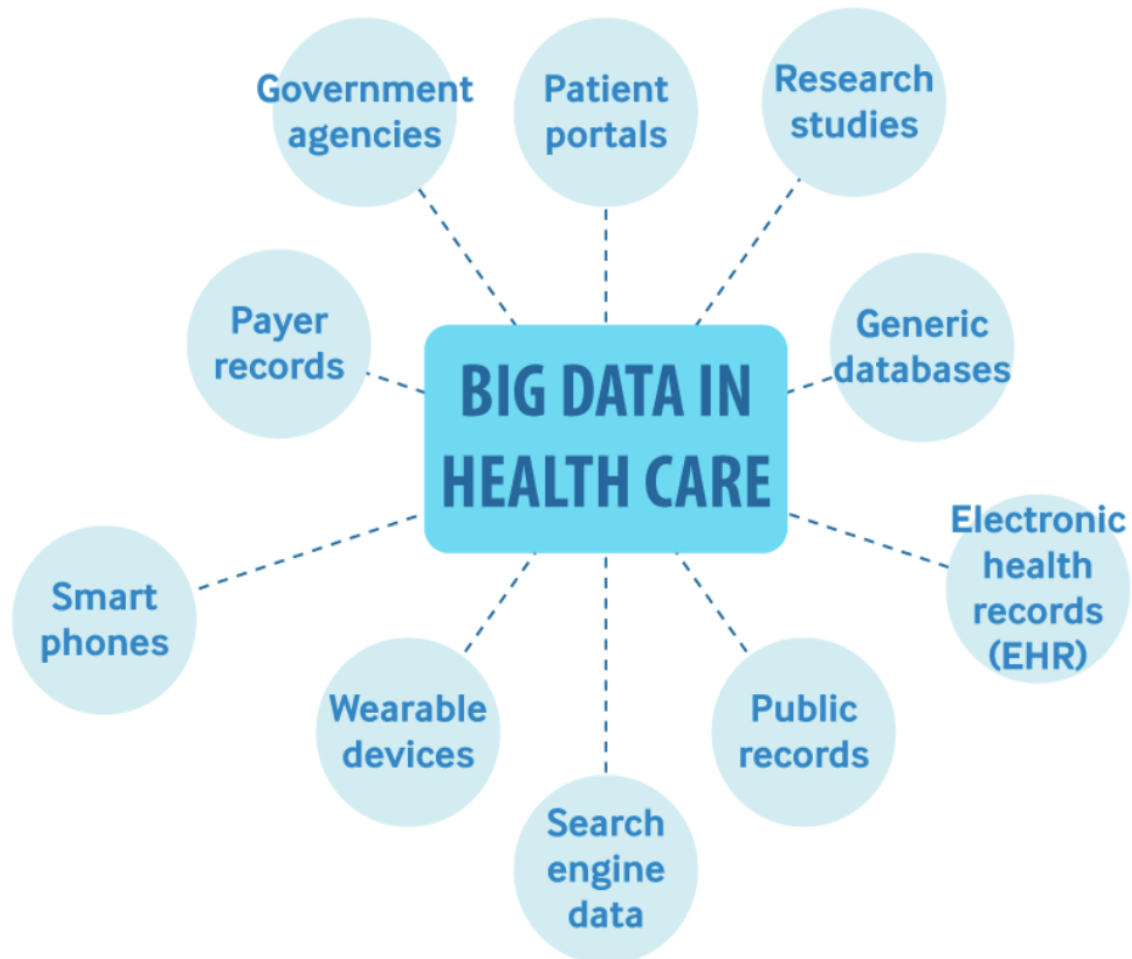
As of March 2020

# Evolving Healthcare Landscape



# Era of Big Data

## Sources of Big Data in Healthcare



## Applications for Big Data in Healthcare



# Leveraging Innovation to Deliver Improved Outcomes

Digital health



Big data



Precision medicine



# Despite Improvements in Health, Disparities and Variation in Care Remain

This Issue Views 90,724 | Citations 22 | Altmetric 4322

## Special Communication

November 26, 2019

## Life Expectancy and Mortality Rates in the United States, 1959-2017

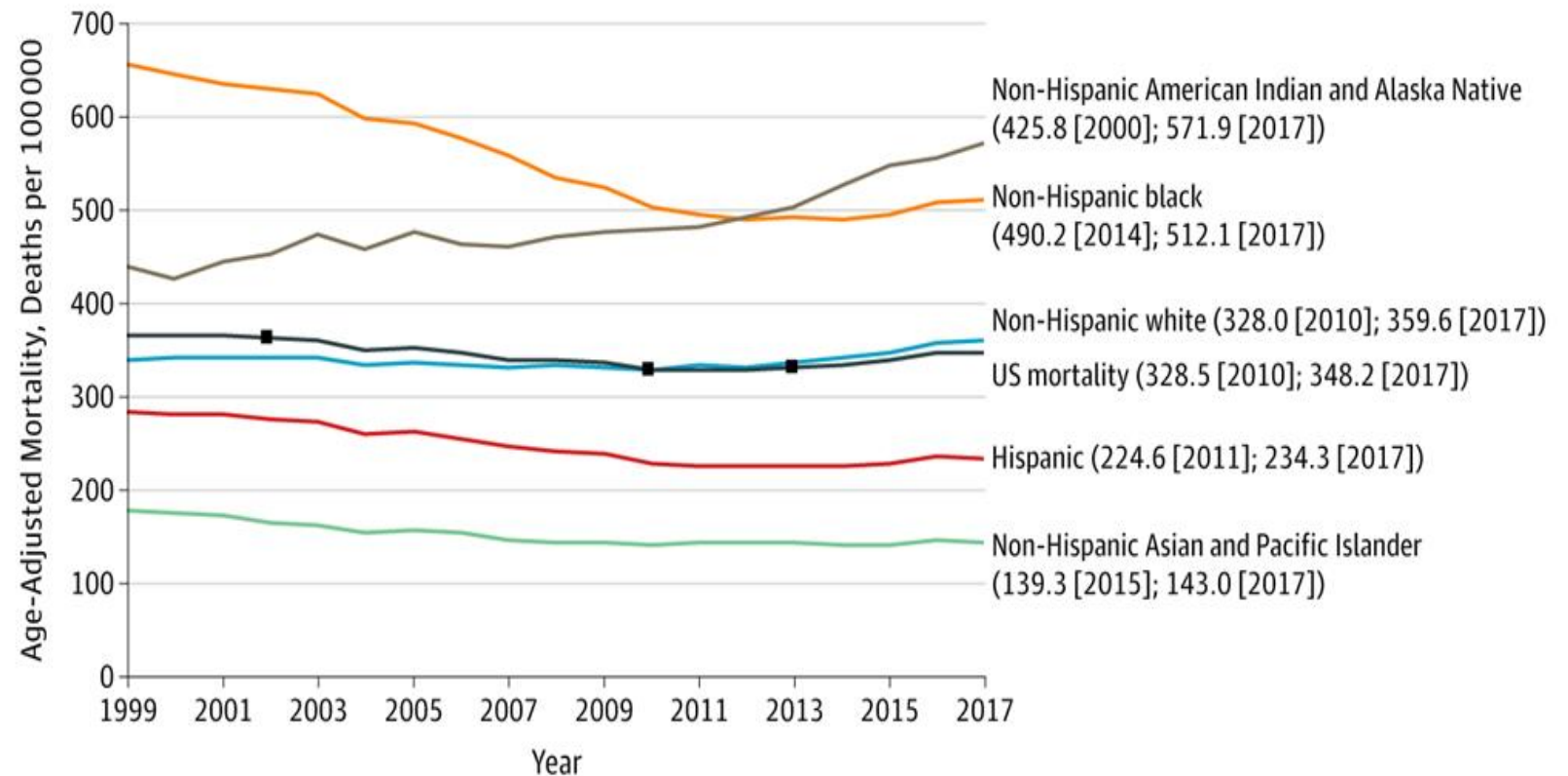
Steven H. Woolf, MD, MPH<sup>1</sup>; Heidi Schoomaker, MAEd<sup>2,3</sup>

» Author Affiliations

JAMA. 2019;322(20):1996-2016. doi:10.1001/jama.2019.16932

Black curve indicates age-adjusted mortality for all US adults aged 25 to 64 years; bolded data points indicate joinpoint years, when the linear trend (slope) changed significantly based on joinpoint analysis. The lowest mortality rates per 100 000 (and the years they were achieved) are listed first in parentheses; mortality rates for 2017 listed second.

Age-Adjusted Mortality Rates, US Adults Aged 25-64 Years, by Race/Ethnicity, 1999-2017



Source: CDC WONDER.

Copyright 2019 American Medical Association.

All Rights Reserved. Date of download: 4/28/20

<https://jamanetwork.com/journals/jama/article-abstract/2756187>

**CORONAVIRUS**  
**USA**

[COVID-19]

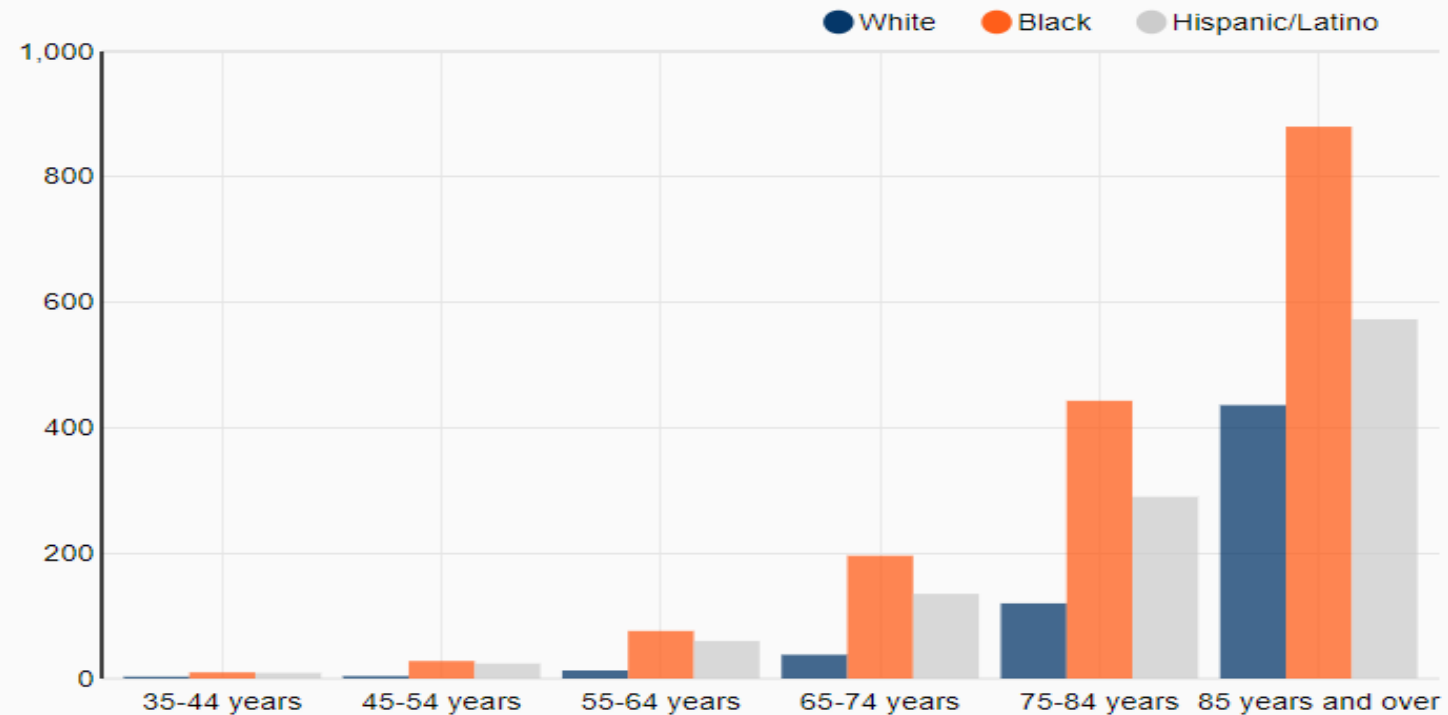
# Health Disparities and COVID-19

# Health Disparities and COVID-19:

## Unmasking the Interplay Between Race & Ethnicity and COVID-19

**Figure 1. COVID-19 death rates by age and race**

Rates per 100,000



Source: CDC data from 2/1/20-6/6/20 and 2018  
Census Population Estimates for USA

BROOKINGS

**Source:** The Brookings Institution. [Race gaps in COVID-19 deaths are even bigger than they appear.](#) COVID-19 death rates by age and race based on CDC data from 2/1/20 – 6/6/20 and 2018 Census Population Estimates for USA. Accessed 7/9/20



# Pandemic of Unprecedented Scale and Consequences

Cumulative  
confirmed  
cases of  
COVID-19

**~34 million  
worldwide**



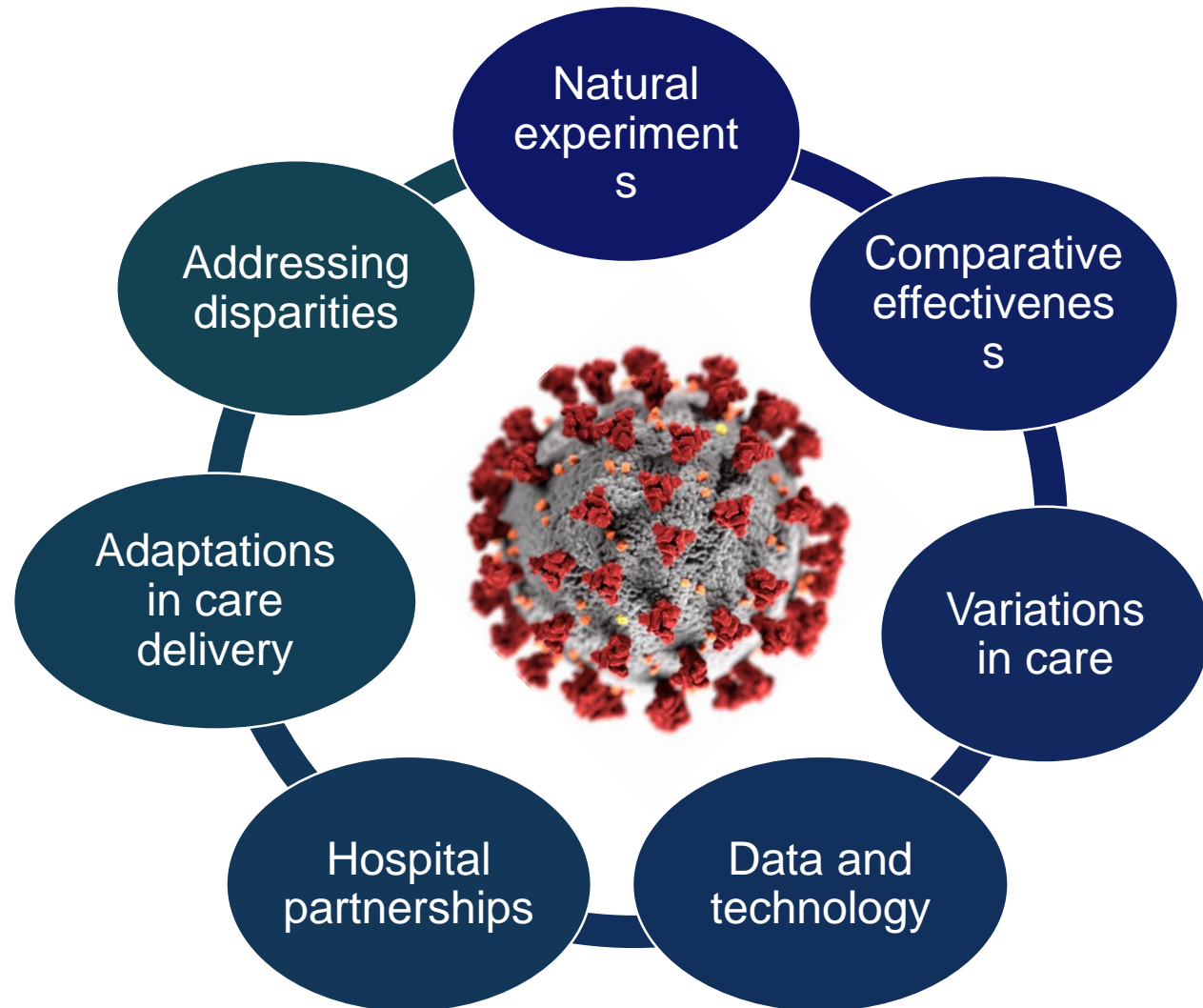
**Source:** COVID-19 Dashboard by the Center for Systems Science and Engineering (CSSE) at Johns Hopkins University (JHU). <https://coronavirus.jhu.edu/map.html> Accessed 9/30/20.



# A Stress Test and Learning Opportunity

## Advancing PCORI 2.0

A Learning  
Organization  
Nimble Embracing  
Opportunities in a  
Crisis for Short and  
Long-term Impact



# PCORI's Response to the COVID-19 Health Crisis

**Healthcare  
Delivery**

**Vulnerable  
Populations**

**Healthcare  
Workers**

**Emerging  
Health Trends**

# PCORI's Response to the COVID-19 Health Crisis

**Healthcare  
Delivery**

**Vulnerable  
Populations**

**Healthcare  
Workers**

**Emerging  
Health Trends**

Many approaches to supporting critical work in these areas and more:

## **Awards**

- Enhancements of existing awards
- Solicitation of new awards
- Healthcare worker registry/trial

## **Information Sharing**

- Horizon Scanning
- Webinars
- Collaboration with other funders

## **Adapting for Awardees and Applicants**

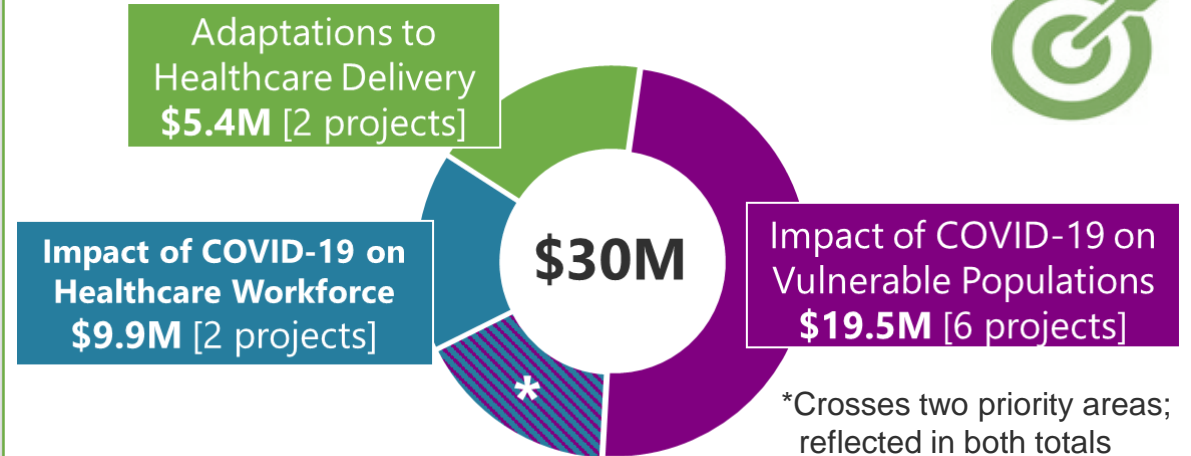
- Adaptations to existing projects
- Extending application timelines

# PCORI COVID-19 Funding to Date

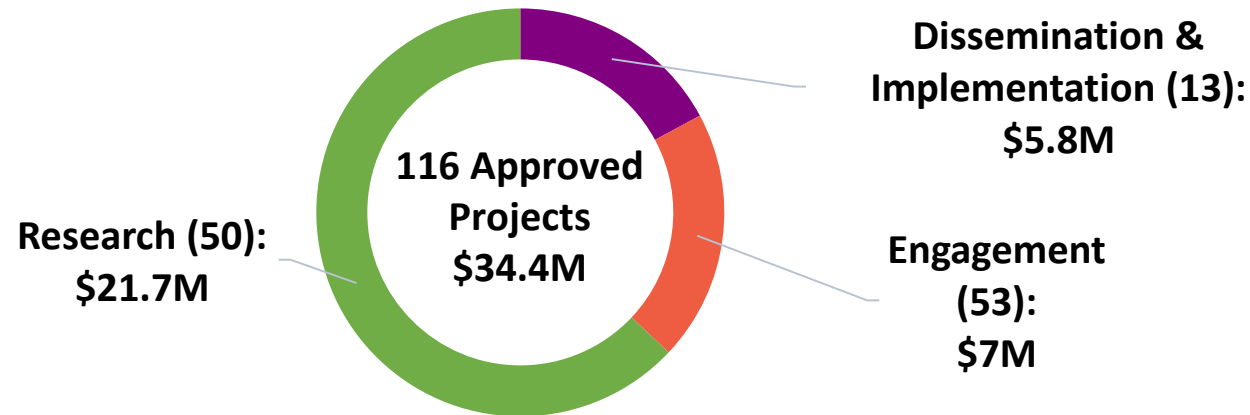


[www.heroesresearch.org](http://www.heroesresearch.org)

## COVID-19 Targeted Funding



## COVID-19 Enhancement Projects





# PCORI – Always Relevant

## *Meaningful research questions in healthcare delivery*



What is the experience of healthcare workers during COVID-19 that can inform key clinical comparative effectiveness questions?

What are the most effective practices for telehealth? Can our current natural experiments shed light on whether telehealth can deliver similar outcomes for patient care and management?

What are the predictors of poor outcomes in COVID-19 infection and can we systematize predictive algorithms to determine who is likely to need intensive care?

What approaches are effective in eliminating health and healthcare disparities in COVID outcomes and how are they generalizable to disparities more broadly?



# PCORI 2.0: The Journey Ahead

# Accelerating Impact on Care and Patient Outcomes

## Engagement

- New and sustained partnerships
- Diverse stakeholder inclusion

## Impacts

- Patients, clinicians, payers, systems as partners for dissemination and uptake
- Evidence to implementation

## Innovation

- Patient-centered learning health care
- Rapid evidence to implementation
- Pragmatic approaches to disparities

## Impacts

- “Real-time” implementation of findings
- Reduced variation in care and disparities in conditions/populations

## Operations

- Creative funding approaches
- Efficient and effective processes

## Impacts

- Nimble, adaptable methods to speed innovation to results to patients
- Robust pool of outstanding research

# Envisioning PCORI 2.0



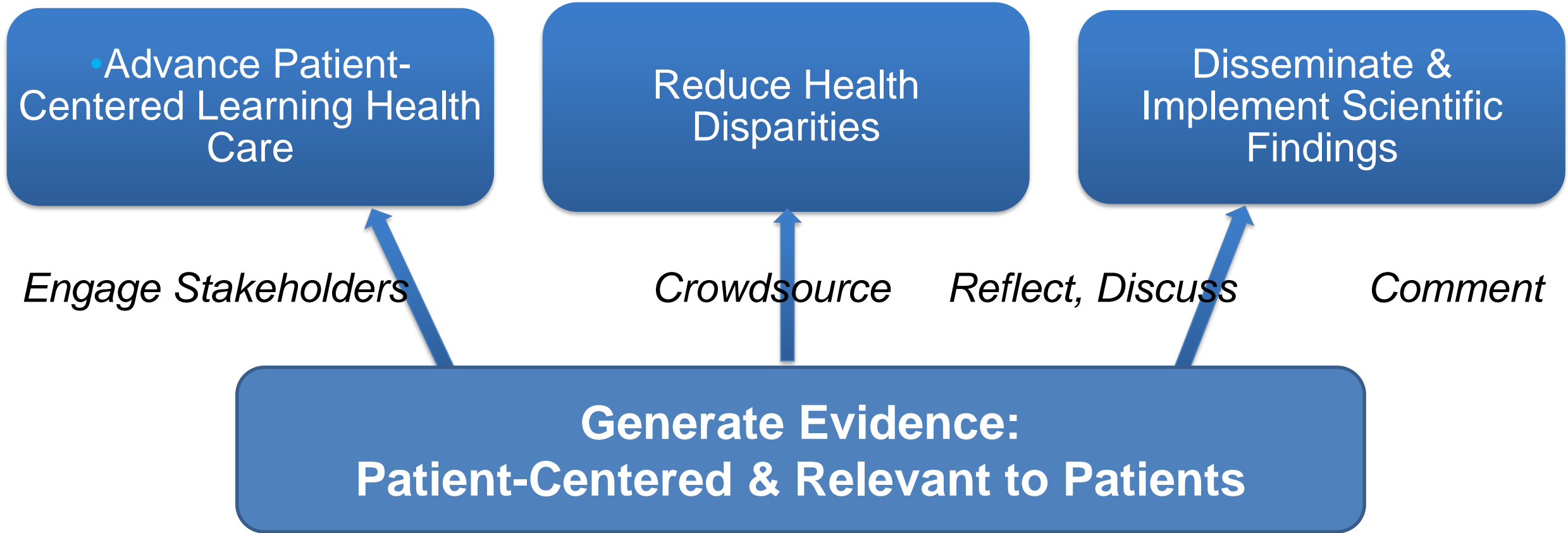
- Advance Patient-Centered Learning Health Care

Reduce Health Disparities

Disseminate & Implement Scientific Findings

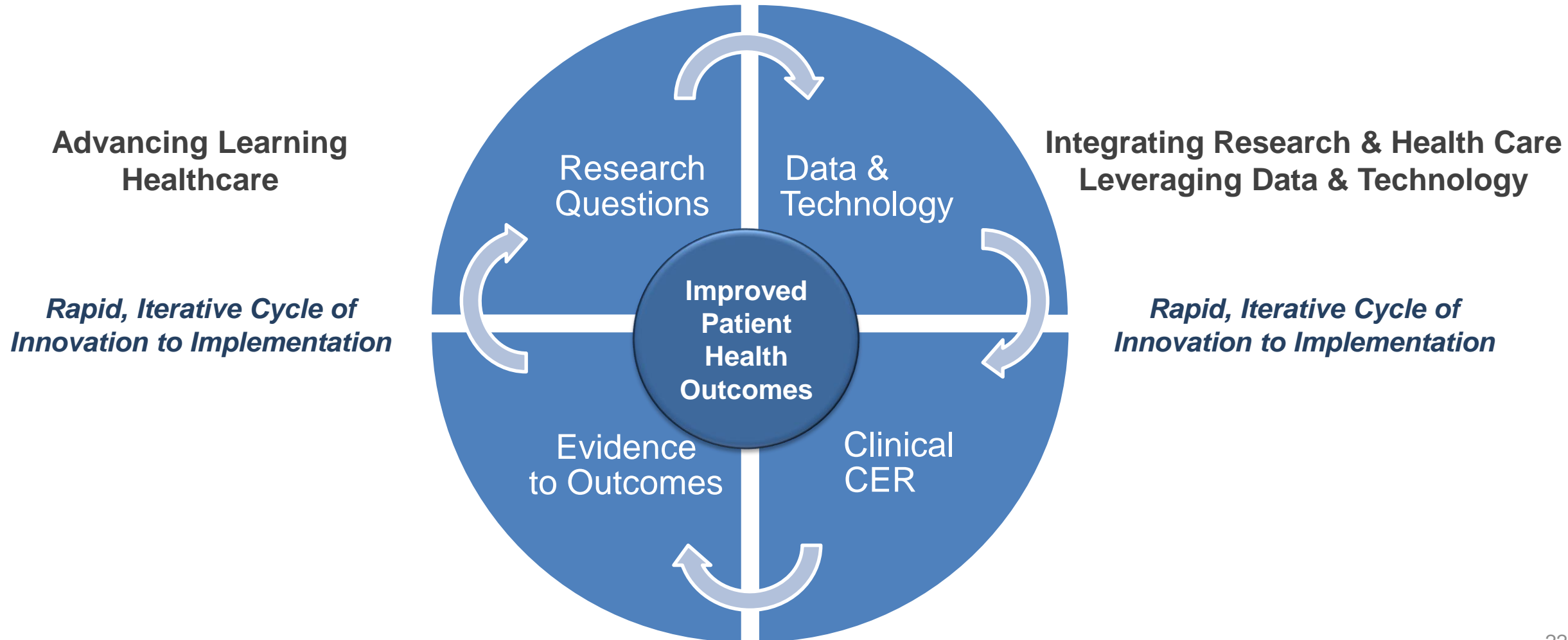


# Envisioning PCORI 2.0



***Pillars underscored by current pandemic***

# PCORI 2.0: A Virtuous Cycle of Evidence to Implementation



# Reauthorization Priorities

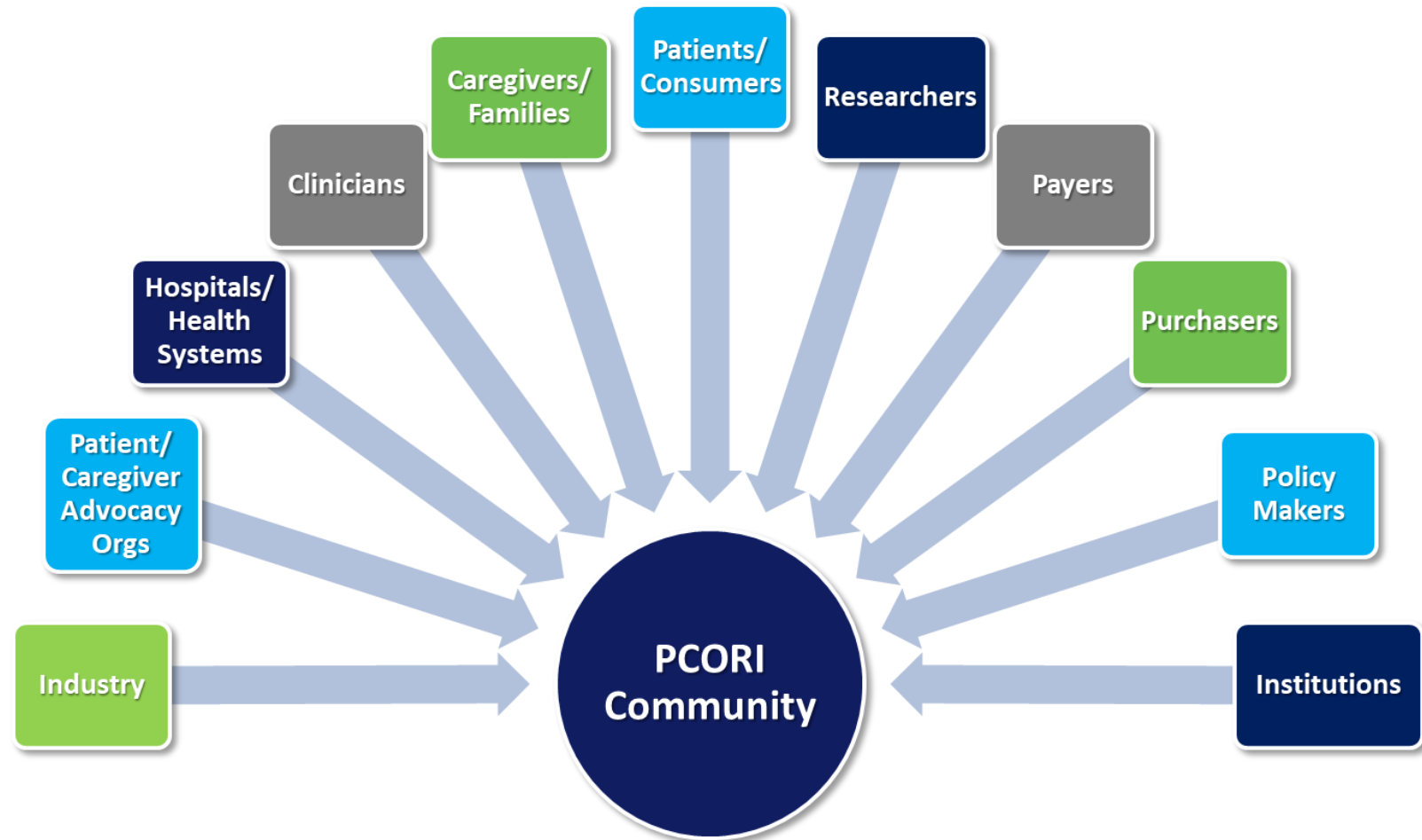
## Strategic Planning

- National priorities frame the research agenda and topic development
- Maternal morbidity and mortality
- Intellectual and developmental disabilities

## Implementing Provision on Cost/Economic Data

- Expanding PCORI's role in collecting and generating relevant evidence
- Focusing on a deliberate and transparent process for implementation

# Collaborative Strategy to Advance a Vision for PCORI 2.0





# Learning From Diverse Stakeholder Groups



Advisory Panels

Payers

Purchasers

Congressional Leaders

Patients

- National Priorities
- Priorities in Legislation

Implementation of  
Cost Data Provision

Data to Support Value  
in Health Care

Addressing Social  
Determinants of  
Health

- Eliminating Disparities
- Priorities in Legislation
- COVID-19 Response

- Patient-Driven Research
- Diversity & Inclusion
- Priorities in Legislation

# Maternal Morbidity and Mortality and Intellectual and Developmental Disabilities

## Stakeholder Engagement

- Has begun
- Long-term
- Existing, new, diverse groups & lived experience

Funding Opportunity Development

Framing of Other Evidence Products (e.g., systematic reviews)

Implementation Plans for Data Collection



The screenshot shows the PCORI website with a navigation bar at the top containing links for BLOG, NEWSROOM, HELP CENTER, SUBSCRIBE, CAREERS, FIND IT FAST, and CONTACT US. Below the navigation bar is a search bar. The main content area features a header for 'Funding Opportunities' and a sub-header for 'Broad PCORI Funding Announcements - Cycle 3 2020 (for Addressing Disparities, Assessment of Options, Communication and Dissemination Research, Improving Healthcare Systems)'. The text below the header states: 'This PCORI Funding Announcement opened on Tuesday, September 1, 2020. Letters of Intent are due Tuesday, September 29, 2020, by 5:00 p.m. ET. Full applications will be due Tuesday, January 12, 2021, by 5:00 p.m. ET.'

## Special Areas of Emphasis:

- Increasing Access to and Continuity of Patient-Centered Maternal Care
- Improving Care for Individuals with Intellectual and/or Developmental Disabilities Growing into Adulthood

# Implementing New Priorities Related to Cost/Economic Data



- PCORI's reauthorizing legislation directs PCORI to capture, as appropriate, the **full range of outcomes data** in our research studies.
- This includes economic and cost impacts **and** patient-centric cost and burdens related to utilization of health care services:
  - Medical out-of-pocket costs, including health plan benefit and formulary design, non-medical costs to patients and families, including caregiving, effects on future costs of care, workplace productivity and absenteeism, and healthcare utilization.

*PCORI is still prohibited from developing QALY measures and conducting cost-effectiveness analysis via our authorizing legislation.*

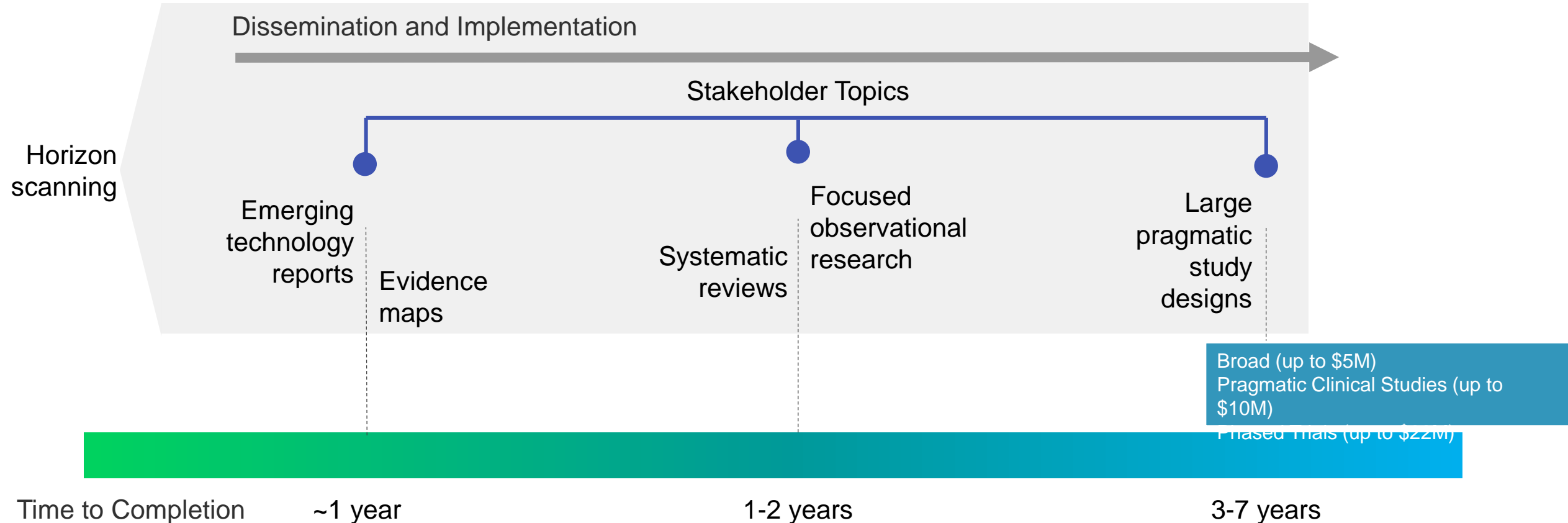




# Comparative Clinical Effectiveness Trials

# Balancing Long- and Short-term Activities

Focused on a range of evidence products to balance long- and short-term results and increasing D&I activities

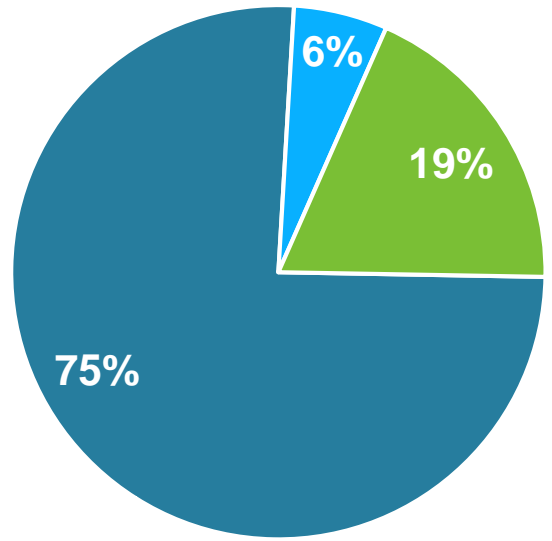




# PCORI Funded CER Portfolio: RCT Snapshot



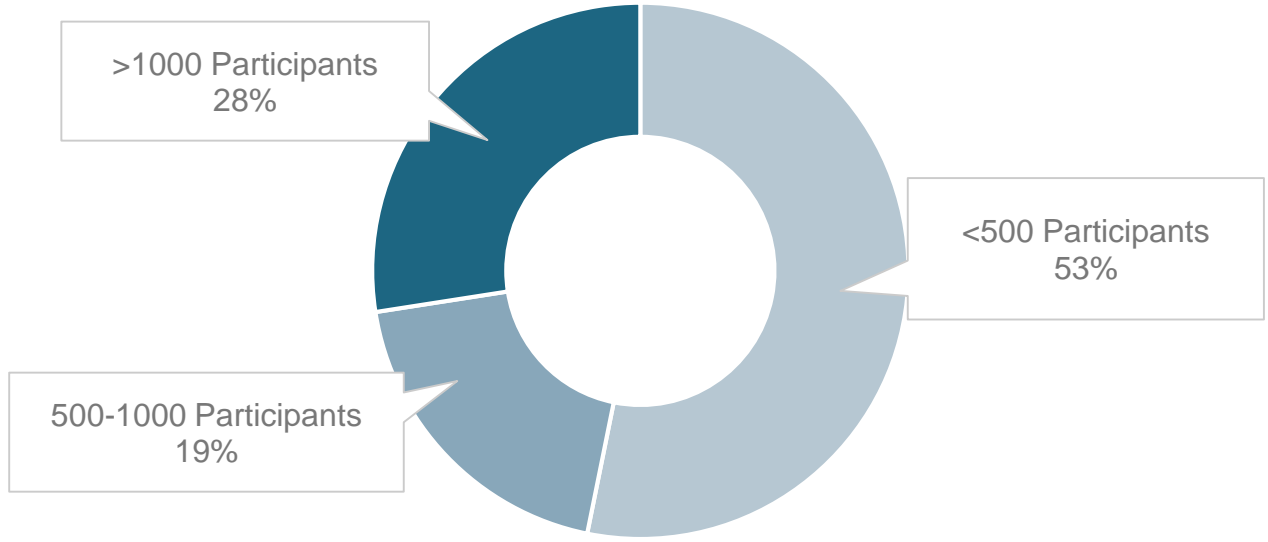
Study Design by  
Number of Awards  
(N=493)



- RCTs
- Quasi-Experimental
- Observational

Design categories are mutually exclusive. Sample includes studies with design measures funded through March 2020.

Target Enrollment:  
RCTs with Individual Enrollment  
(N=346)



**Median:** 449 participants per study  
**Average:** 1334 participants per study

25% of PCORI-funded RCTs have a Cluster Randomized Design

# New Funding for Large Pragmatic Trials

Phased Large Awards for Comparative Effectiveness Research (PLACER)



**Objective:** Support large-scale, high-impact CER trials that examine critical patient-centered research questions also relevant to decision makers and other stakeholders through a phased approach to optimize achievement of research aims

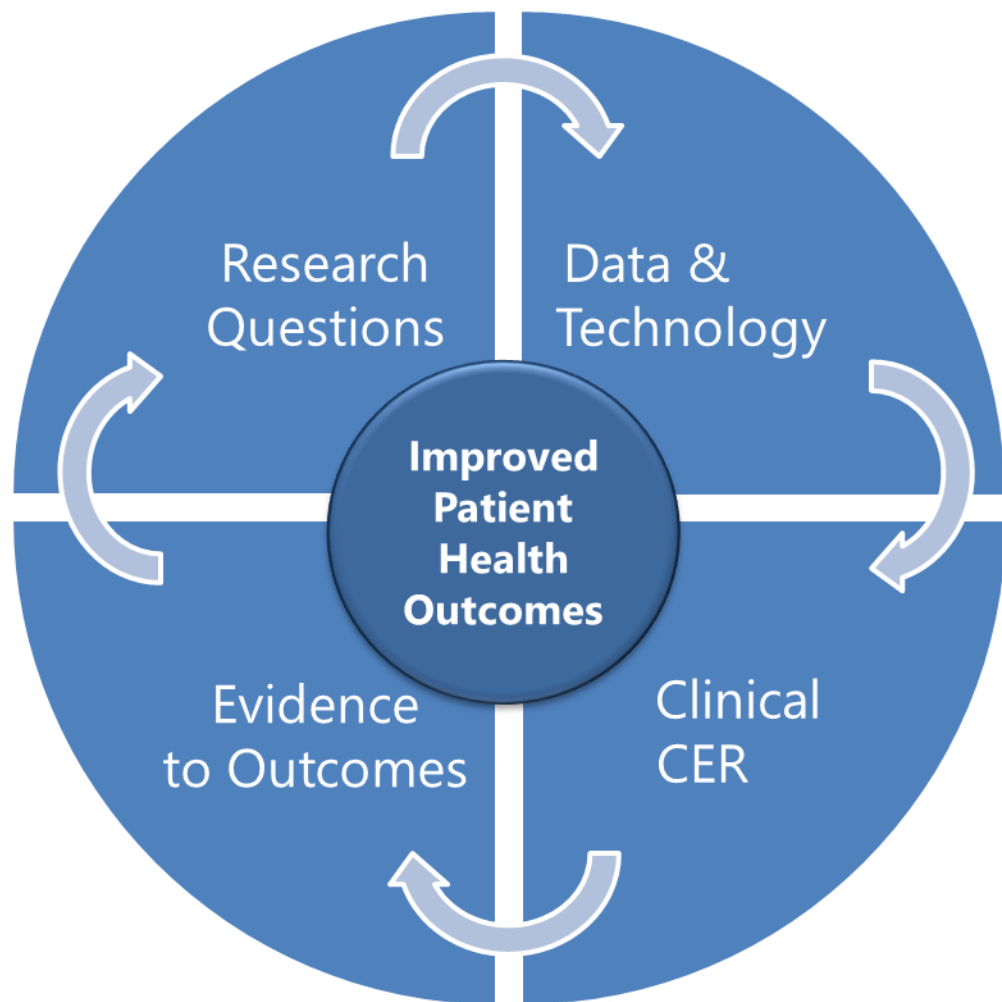
## Exemplar questions:

What are the comparative benefits and/or harms for different systolic blood pressure targets in older adults with hypertension and comorbid conditions on outcomes such as adverse events and cognitive impairment?

What is the comparative effectiveness of culturally tailored care models to improve maternal outcomes (including patient access, continuity of care, and patient experience) and reduce disparities, morbidity, and mortality?

**Available funds:** \$150 million

# Comparative Clinical Effectiveness Trials: Opportunities Ahead



Digital health



Big data



Precision medicine

Adapted from Bhavnani et al. *J Am Coll Cardiol*. 2017;70:2696–718

- Integrating Trials into Clinical Care
- Leveraging Wearables, Big Data: AI & ML, Technology for Outcomes
- Accelerating Translation and Impact with Engagement, Partnership, and Planned D&I

# Where We Go from Here

*Imagine a day...*



*When most patients prescribed a new therapeutic or device without evidence of superiority or non-inferiority (clear equipoise) are randomized to relevant comparative strategies,...*



*When patient-reported and health outcomes are collected routinely and digitally recorded for future analyses that are done efficiently and regularly,...*



*And when patients, providers, and policymakers have equal access to the evidence to make informed choices, recommendations, and policy regardless of who the patient is, where they live, or their socioeconomic status.*

***The opportunities are ours to seize!***

# Thank You

