PCORI Advisory Panel on Healthcare Delivery and Disparities Research

Spring 2020 Meeting

June 11, 2020

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Housekeeping

- Webinar is available to the public and being recorded.

- Members of the public are invited to listen to this teleconference and view the webinar.

- Meeting materials will be posted on the PCORI website after the meeting.

- Anyone may submit a comment through the webinar chat function, although no public comment period is scheduled.

- Visit www.pcori.org/events for more information.

- Statement on COIs and confidentiality

1.

Welcome and Introductions

Panel Leadership

- **Nadine Barrett, PhD, MA, MS**
  HDDR Advisory Panel Co-Chair

- **Frank Wharam, MD, MPH**
  HDDR Advisory Panel Co-Chair

- **Steve Clauser, PhD, MPA**
  Director, Healthcare Delivery and Disparities Research Program

HDDR Advisory Panel Members

Patient/Caregiver Advocate or Advocacy Organization

- **Barbara Warren, Psy.D**
  Director for LGBT Programs and Policies in the Office for Diversity and Inclusion, Mount Sinai Health System and Assistant Professor of Medical Education

- **Carmen Pace, BSN, LPN, MPA, RN**
  Facing Our Risk of Cancer Empowered (FORCE)

- **Mary Grace Pagaduan, MPH**
  March of Dimes Foundation

- **Kathy Phipps**
  Community Health Worker, Memorial Hermann Health System

- **Ana Lopez, BSN, RN**
  Facing Our Risk of Cancer Empowered (FORCE)

Hospital/Health Systems

- **Xiaoduo Fan, MD**
  Associate Professor, Psychiatry, University of Massachusetts Medical School

Industry

- **Danielle Brooks, JD**
  Senior Consultant and Director of Patient Engagement, WiseThink Health Solutions; Founder & CEO, Bridges

Clinicians

- **Jennifer Potter, MD**
  Professor, Beth Israel Deaconess Medical Center

- **Kathleen Kieran, MD, MSc, MME**
  Physician, Seattle Children's Hospital

HDDR Advisory Panel Members

Researchers

- **Alicia Arbaje, PhD, MD, MPH**
  Associate Professor of Medicine and Director of Transitional Care Research, Johns Hopkins University

- **Cheryl Holly, EdD, MED, RN**
  Professor, Rutgers School of Nursing

- **Rainu Kaushal, MD, MPH**
 Professor of Healthcare Policy and Research, New York-Presbyterian Hospital

Payers

- **Marissa D. Sanders, MPH, CPHRM**
  Manager, Quality Assessment, American Dental Association

- **Thomas James, III, MD**
  Chief Medical Officer, WellCare/Centene of Kentucky

- **Jane Kogan, PhD**
  Associate Chief Research and Translation Officer, UPMC Insurance Services Division and Center for High-Value Health Care

- **Rachel Raia, MPH**
  Director of Data Analytics, Ascension Health

Healthcare Delivery and Disparities Research Staff

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Steve Clauser, PhD, MPA
Program Director
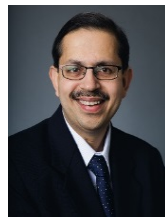
Neeraj Arora, PhD
Associate Director

Els Houtsmuller, PhD
Associate Director
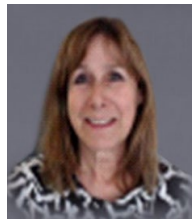
Penny Mohr, MA
Senior Advisor

Carly Parry, PhD, MSW
Senior Advisor

Allison Ambrosio, MPH
Sr. Program Manager

Beth Kosiak, PhD
Program Officer

Mari Kimura, MS, PhD
Program Officer

Andrea Brandau, MPP
Program Officer

Carly Khan, PhD, MPH, RN
Program Officer

Vivian Towe, PhD, MSc, MA
Program Officer

Cathy Gurgol, MPH
Sr. Program Officer

Sindhura Gummi, MPH
Sr. Program Associate

Soknorntha Prum, MPH
Sr. Program Associate

Juliette Price, MPH
Program Associate

Brendan Weintraub, MPH
Program Associate

Candace Hall, MA
Program Associate

Tshema Nash, MPH
Program Associate

Aaron Shifreen
Program Associate

Metti Duressa
Program Associate

Rachel Kotiah
Sr. Administrative Assistant

Elizabeth Zofkie, MPH
Program Associate

Welcome New HDDR Staff

Vivian Towe, PhD, MSc, MA
Program Officer

2.

Agenda

Agenda

(Times in Eastern)

- 11:30 AM HDDR Program Updates

- 12:30 PM Welcome from PCORI Executive Director

- 1:15 PM Break

- 1:45 PM National Priorities and Research Agenda

- 2:30 PM Overview of PCORI Reauthorization

- 3:00 PM Break

- 3:15 PM Cost Data Provision

- 4:30 PM Wrap-Up

3.

Healthcare Delivery and Disparities Research Program Updates

Steven Clauser, PhD, MPA

Director, Healthcare Delivery and Disparities Research Program

HDDR Updates

- New Research Awards
- Research and Learning Networks
- Update on Priority Initiatives
- Research in the Pipeline

New Research Awards

Current Portfolio

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PCORI HAS AWARDED OVER

$990 **MILLION** **TO FUND** **234**

COMPARATIVE EFFECTIVENESS STUDIES IN HEALTHCARE DELIVERY AND DISPARITIES RESEARCH.

As of April 2020

Funding Mechanism	# of Projects
Broad	177
Pragmatic	21
Targeted	36

New Research Awards

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Recent Awards

4 Improving Healthcare Systems Broad Awards

2 Addressing Disparities Broad Awards

*AWARDED January 2020 & March 2020

New Broad Awards:
Improving Healthcare Systems

Study Title	PI Name	Site
Comparative Effectiveness of Readmission Reduction Interventions for Individuals with Sepsis or Pneumonia	Namita Ahuja Yende	UPMC Center for High-Value Health Care
Pediatric Trauma Care Models' Impact on Family Experience, Traumatic Stress, and Financial Hardship	James Marcin	UC Davis
Comparative Effectiveness of Perinatal Psychiatric Access Programs on Treatment Engagement	Thomas Mackie	Rutgers Biomedical and Health Sciences
Comparative Effectiveness of Mobile Integrated Health versus Chronic Care Model for Post-Discharged Heart Failure Patients	Rainu Kaushal	Weill Medical College of Cornell University

New Broad Awards:
Addressing Disparities

Study Title	PI Name	Site
Examination of the Evidence-Based Care Transitions Intervention Enhanced with Peer Support to Reduce Racial Disparities in Hospital Readmissions and Negative Outcomes Post Hospitalization	Kyaien Conner	University of South Florida
Comparing Mobile Health Strategies to Improve Pre-exposure Prophylaxis Use (PrEP) for HIV Prevention	Albert Liu	Public Health Foundation Enterprises, Inc

Examining Our Portfolio – Cancer Care

- Board of Governors Presentation on December 2, 2019
  - Overview of PCORI's Cancer CER Portfolio
  - Study Findings to Date
  - Summary and Next Steps
- Desire to consider new stakeholder-driven topics

Studies Address Decisional Dilemmas Across the Patient's Cancer Journey

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Prevention and Early Detection

- **Uptake in priority populations**
- Risk-based approaches to screening
- Communication and decision-making

Treatment

- **Comparison of treatment options**
- Symptoms and side effects
- Caregiver burden
- Models of care
- Communication and decision-making

Survivorship

- **Post-treatment surveillance**
- Symptoms and side effects
- Caregiver burden
- Models of care

Examining Our Portfolio – Community Health Workers

Board of Governors Presentation - March 2, 2020

- Overview of PCORI's Community Health Worker (CHW) Portfolio
  - Complexities in reducing health and healthcare disparities
  - Understanding the role of CHWs
  - Study spotlights across PCORI's CHW portfolio
- PCORI Awardee Presentations
  - Raj Shah, PhD and Kevin English, DrPH, University of New Mexico
    - *Reducing Health Disparity in Chronic Kidney Disease in Zuni Indians*
    - *Home-Based Chronic Kidney Disease (CKD) Care in Native Americans of New Mexico: A Disruptive Innovation*
  - Shreya Kangovi, MD, University of Pennsylvania and Keysha Brooker, MSW
    - *Effectiveness of Collaborative Goal-Setting Versus IMPaCT Community Health Worker Support for Improving Chronic Disease Outcomes*
    - *Implementation of the IMPaCT Community Health Worker Intervention*

Research and Learning Networks

Research and Learning Networks

- Transitional Care Evidence to Action Network (TC-E2AN)

- Palliative Care Learning Network

- Telehealth Research Portfolio Synthesis Group
  - Discussion on Telehealth research in COVID era – Penny Mohr

Transitional Care Evidence to Action Network (TC-E2AN)

- 3 new studies have been added, for a total of 29 studies, ~$128 million investment

- Hosted webinar addressing methodological challenges caused by COVID-19 interruptions

- *Medical Care* approved a Special Issue based on a prospectus highlighting work from the TC-E2AN.
  - Targeted release: Summer 2021
  - Includes commentaries, an editorial, several composite topic-driven papers, and papers from individual studies

- Generating summary report from the 5 years of the network

- Virtual webinar meeting may occur this fall

Palliative Care Learning Network

- HDDR has created a learning network of nine multi-site palliative care CER studies that were funded in FY2017 for a total investment of ~$81 million
  - Six studies focus on models of palliative care delivery and three focus on advance care planning

- Network goals: foster co-learning across awardee teams; facilitate collective success of the projects; contribute to the field via joint presentations and publications

- 2020 Network activities:
  - Advance care planning awardees were accepted for a joint panel presentation at the American Academy of Hospice and Palliative Medicine Annual Assembly in March 2020
  - 4th annual in-person awardee meeting being planned for Fall 2020
  - Ongoing conference calls facilitated by HDDR among investigators and project managers

- Progress of these studies will be summarized in an annual report in June 2020

Telehealth Research Portfolio Synthesis Group

- Large and diverse portfolio; current investment is 94 studies amounting to ~$400 million
- Supporting ongoing manuscripts led by investigators on the following topics:
  - Addressing Disparities through Telehealth
  - Examining Evidence Gaps in the Use of mHealth for Self-Management of Chronic Disease
  - Challenges in Addressing Large, Multi-site, Multi-state Pragmatic Trials in Telemedicine
- Cross-PCORI COVID-19 Telehealth Work Group
  - Goal: What is shifting in the field of telehealth in relation to the COVID-19 pandemic and how does that alter the way we think about future investment in this area moving forward?
    - Evaluate telehealth project enhancements, adaptations, and newly funded projects under COVID-19
    - Understand evidence gaps around telehealth innovations (e.g., primary and urgent care)
    - Communicate to outside stakeholders' findings and lessons learned that may be relevant to groups rapidly implementing telehealth

Update on Priority Initiatives

- Suicide Prevention
  - Iterative topic prioritization with stakeholders
  - Brief Interventions for Youth (ages 15-24) identified as high priority topic
- Congressional mandates
  - Maternal Mortality
    - Significant interest in research questions regarding maternal care coordination, patient/provider education, and wrap around services
    - Ongoing engagement and research to refine CER questions related to obstetrical emergencies and perinatal mental health
  - Intellectual and Developmental Disabilities
  - Ongoing engagement to identify gaps and refine research topics
    - Interest in research questions related to transition to adulthood, treatments across the life course, and care delivery

In the Pipeline

Upcoming Targeted Research Opportunities

COVID-19 Targeted PFA

- Up to $30M funds available
- PFA Posted: May 2020
- Awards to be Announced: **July 2020**

Targeted Priority Areas:
- Adaptations to health care delivery
- COVID-19 impact on vulnerable populations
- Impact of COVID-19 on healthcare workforce

Upcoming Broad Research Opportunities

2019 Cycle 3 Broad PFA

- Addressing Disparities: Up to $4M funds available
- Improving Healthcare Systems: Up to $8M funds available
- PFA Posted: September 2019
- Awards to be Announced: **July 2020**

2020 Cycle 1 Broad PFA

- Addressing Disparities: Up to $8M funds available
- Improving Healthcare Systems: Up to $16M funds available
- PFA Posted: January 2020
- Awards to be Announced: **November 2020**

Phased Large Awards for Comparative Effectiveness Research (PLACER), Cycle 3 2020

- **Purpose**: To foster large, meritorious, and innovative studies with some risks, increase PCORI funding that directly compares clinical options, and improve success by managing risks of large research investments

- **PLACER is a new and distinctive addition** to the existing PCORI funding suite of Pragmatic Clinical Studies (PCS), Targeted studies, and Broad studies in:
  - Needing larger scale and scope to answer critical research question
  - Having residual uncertainties with non-trivial risks for full, up-front funding
  - Having sufficient evidence and stakeholder interest to warrant investment in further refinement before full-scale execution

PFA Timeline	Dates
PFA release	6/9/20
LOIs due	9/29/20
Applications due	1/12/21
Awards announced	July 2021

Funding	Amount
Feasibility phase	$\leq$ $2M direct costs
Full-scale study phase	$\leq$ $20M direct costs
Funds available	$150M

2020 PCORI Annual Meeting

pcori ANNUAL MEETING

September 16-17, 2020

Virtual Meeting

Navigating Our Course Together

- **My First Few Months at PCORI**
  - My Personal Journey
  - Early Onboarding
  - Passionate and Mission-Driven PCORI Staff and Board

- **Envisioning PCORI 2.0**
  - Opportunities for the Future
  - Learning in a Public Health Crisis

- **Our Course Together**
  - Year 1 Priorities

My Journey: Fulfilling a Life's Purpose

Motivated by CV Disparities in My Neighborhood

University of Alabama at Birmingham

Inspired to Leadership in Health

Harvard Medical School

Defined Patient-Centered Health Care

Massachusetts General Hospital

Pursued Health Services Research

Harvard School of Public Health

Focused on Research Agenda & Management

NHLBI Office of the Director

Privileged to Serve; Destined to Be

Patient-Centered Outcomes Research Institute

Outstanding, Resilient Staff and Passionate, Mission-Driven Panels and Committees at the Heart of PCORI

- **Overcoming Challenges in Pursuit of the Mission**
  "Courage is simply doing whatever is needed in pursuit of the vision"
    — Peter M. Senge, The Fifth Discipline: The Art & Practice of The Learning Organization

- **Dedicated and Mission-Driven**
  - Integrated conceptual framework for AD and IHS
  - Assessed Comparative Effectiveness Research gaps to Promote Aging in Place framework
  - Identified research gaps in PCORI's Mental Health Portfolio
  - Provided recommendations and considerations for Maternal Mortality and Suicide Prevention CER

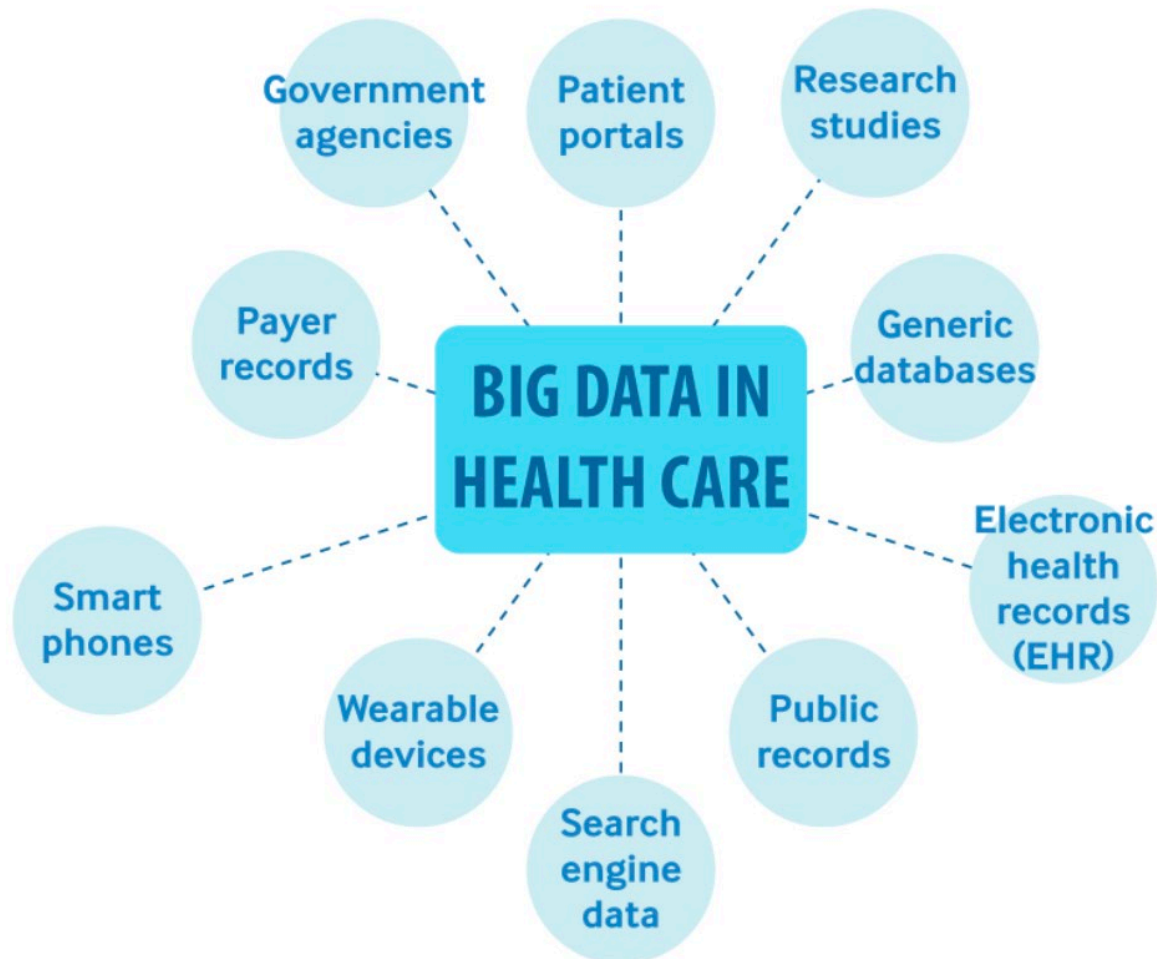 **A true testament to your efforts**

Navigating Our Course Together

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Evolving Healthcare and Research Landscape in a Big Data Era

Sources of Big Data in Healthcare

**Applications for Big Data in Healthcare**

- Diagnostics
- Preventive Medicine
- Precision Medicine
- Medical Research
- Reduction of adverse events
- Cost reduction
- Population Health

Adapted from https://catalyst.nejm.org/big-data-healthcare/
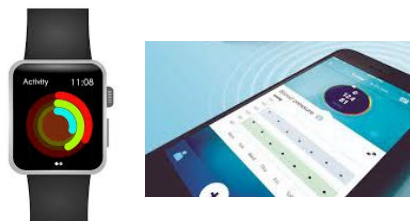
Leveraging Innovations in Healthcare to Promote the Science of Delivery for Improved Outcomes

Bhavnani et al. *J Am Coll Cardiol.* 2017;70:2696–718
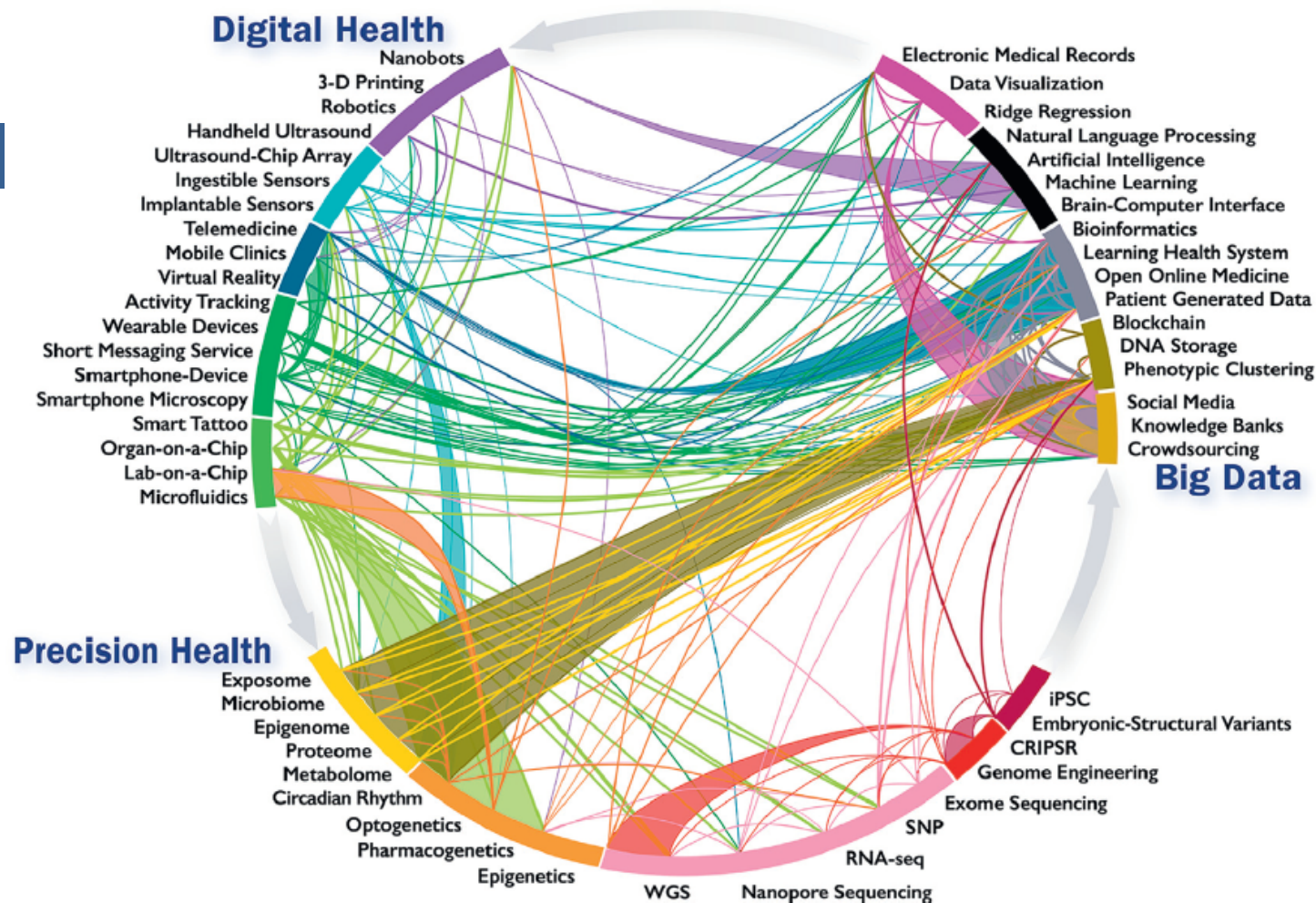
Despite Marked Improvements in Health, Disparities and Variation in Care Still Remain
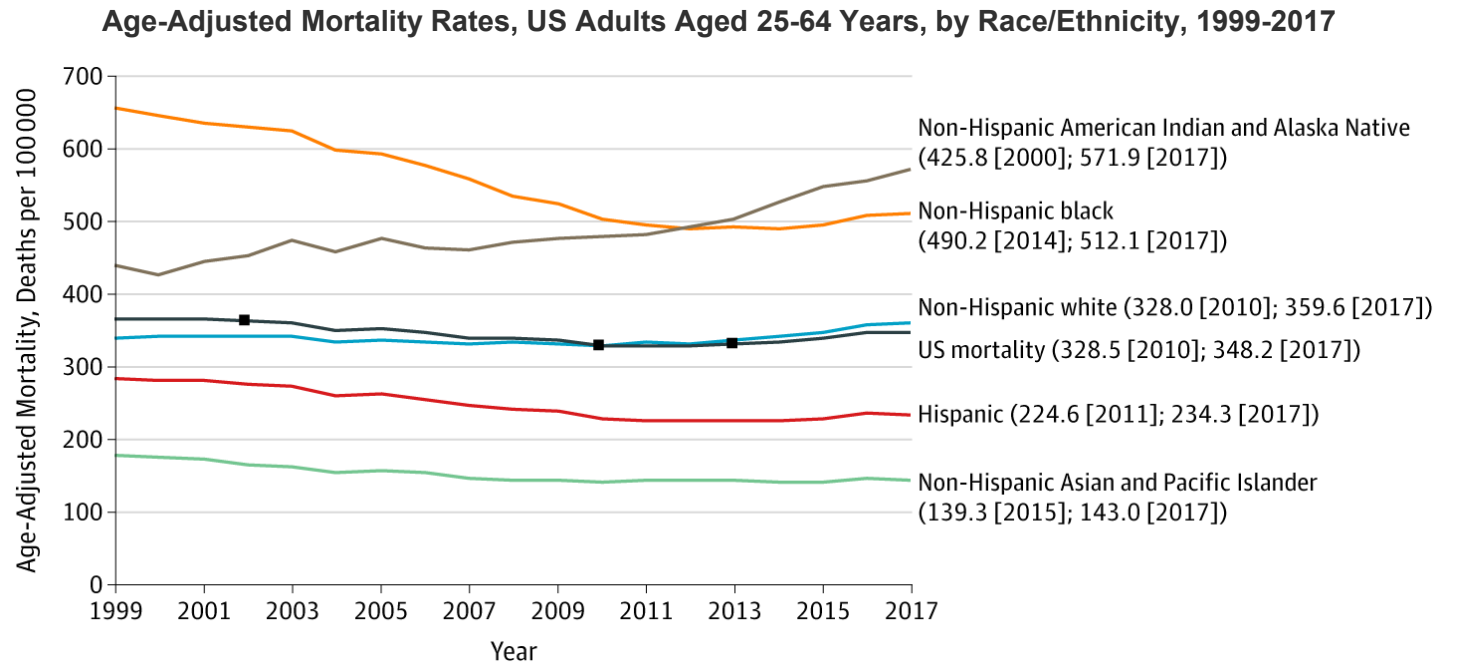
Special Communication

November 26, 2019

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

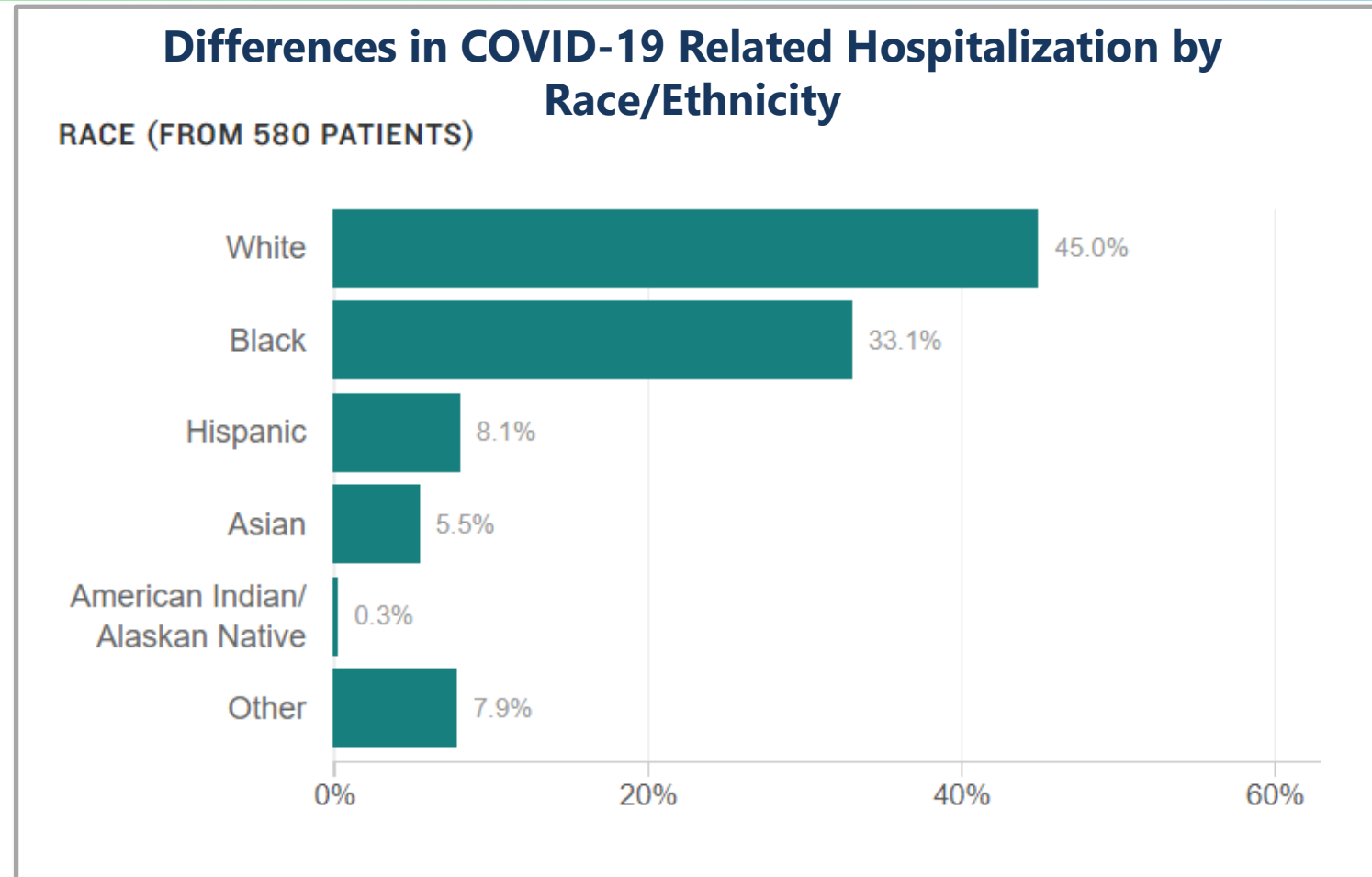Steven H. Woolf, MD, MPH[1]; Heidi Schoomaker, MAEd[2,3]

» Author Affiliations

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

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

- Non-Hispanic American Indian and Alaska Native (425.8 [2000]; 571.9 [2017])
- Non-Hispanic black (490.2 [2014]; 512.1 [2017])
- Non-Hispanic white (328.0 [2010]; 359.6 [2017])
- US mortality (328.5 [2010]; 348.2 [2017])
- Hispanic (224.6 [2011]; 234.3 [2017])
- Non-Hispanic Asian and Pacific Islander (139.3 [2015]; 143.0 [2017])
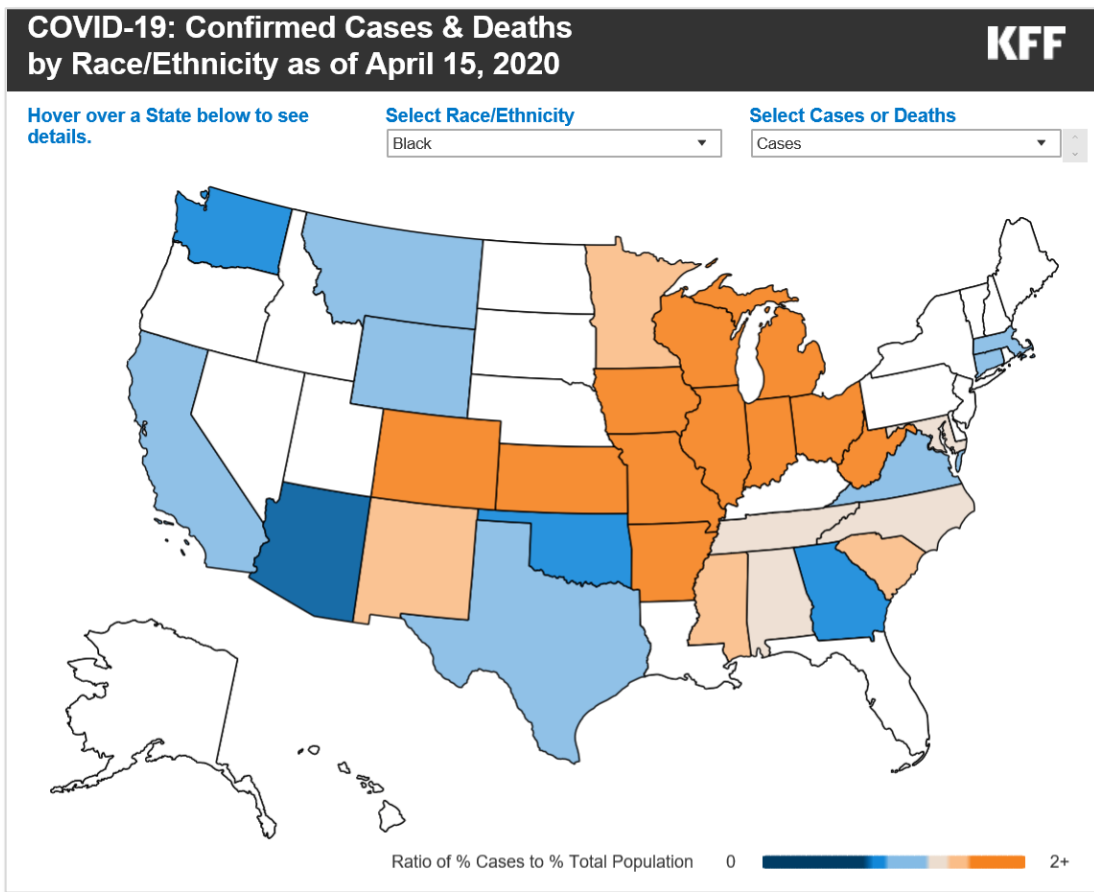
Black curve indicates age-adjusted mortality for all US adults aged 25 to 64 years; bolded data points indicate joinpoint years, when the linear trend (slope) changed significantly based on joinpoint analysis. The lowest mortality rates per 100 000 (and the years they were achieved) are listed first in parentheses; mortality rates for 2017 listed second. Source: CDC WONDER.

COVID-19 Outcomes Clearly Elucidate the Challenge of Health Disparities

Differences in COVID-19 Related Hospitalization by Race/Ethnicity

RACE (FROM 580 PATIENTS)

- White: 45.0%
- Black: 33.1%
- Hispanic: 8.1%
- Asian: 5.5%
- American Indian/Alaskan Native: 0.3%
- Other: 7.9%

Emerging Data: Unmasking the Interplay Between Race/Ethnicity and COVID-19

Source: Kaiser Family Foundation. COVID-19: Confirmed Cases & Deaths by Race/Ethnicity as of April 15, 2020. Distribution of Cases/Deaths by Race/Ethnicity based on KFF analysis of publicly available state websites. Total State Population Distribution by Race/Ethnicity based on KFF analysis of 2018 American Community Survey. Accessed 4/28/20

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A Pandemic of Unprecedented Scale and Consequences

Cumulative Confirmed Cases of COVID-19:

>7 million worldwide
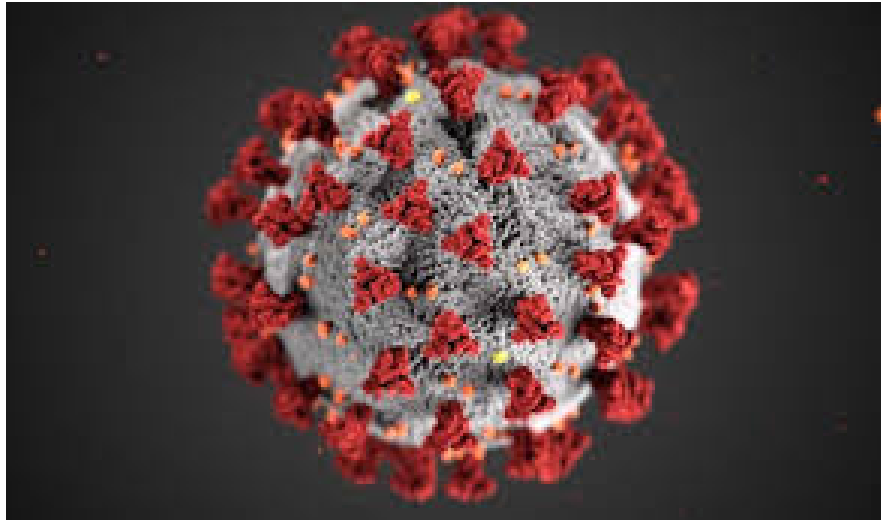
Source: COVID-19 Dashboard by the Center for Systems Science and Engineering (CSSE) at Johns Hopkins University (JHU). https://coronavirus.jhu.edu/map.html Accessed 4/28/20.

COVID-19 Pandemic – Not Only a Stress Test, But Also Learning Opportunity

Advancing PCORI 2.0
A Learning Organization Nimbly Embracing Opportunities in a Crisis for Short and Long-term Impact

- Natural Experiments
- Comparative Effectiveness
- Variations in Care
- Data and Technology
- Hospital Partnerships
- Adaptations in Care Delivery
- Addressing Disparities
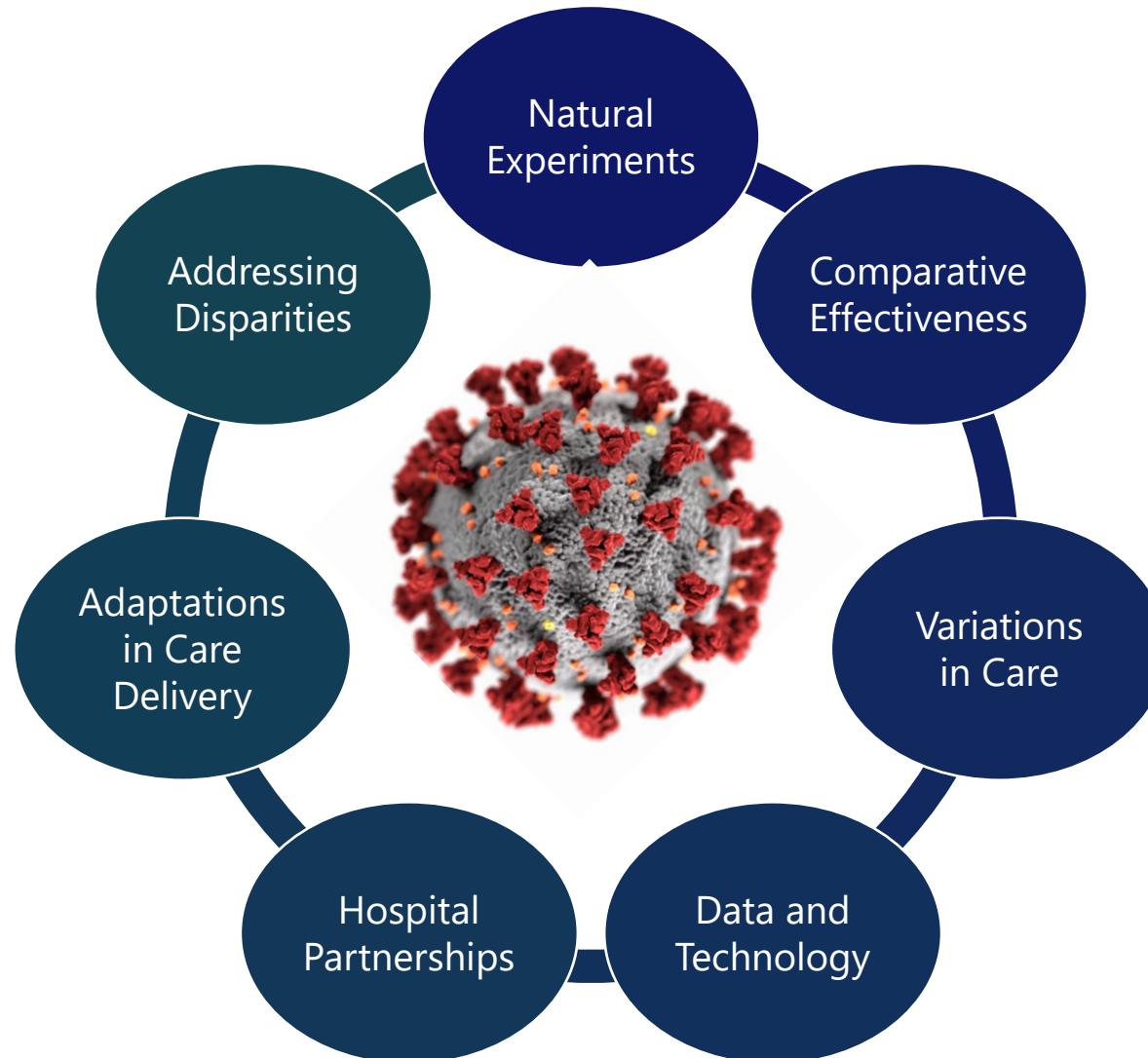
PCORI's Multi-Pronged Approach in Response to the COVID-19 Health Crisis

In our efforts to inform the current pandemic and learn for the future, PCORI has identified **three priority areas**:

Health Care Delivery

Vulnerable Populations

Health Care Workers

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

Awards
- Enhancements of existing awards
- Solicitation of new awards, e.g., natural experiments
- Health care worker registry and trial

Information Sharing
- Webinars
- Collaboration with other funders

Adapting for Awardees and Applicants
- Adaptations to existing projects
- Extending application timelines

HERO
Healthcare Worker Exposure Response & Outcomes
Uniting our healthcare community to protect the health and well-being of America's frontline workers

COVID-19 Enhancements – Current PCORI Funding

Funded Enhancements Data

Last updated: June 10, 2020

D&I (7): **19%**

Engagement (18): **17%**

46 Approved Projects

Research (21): **64%**

- ■ Funded D&I
- ■ Funded Engagement
- ■ Funded Research
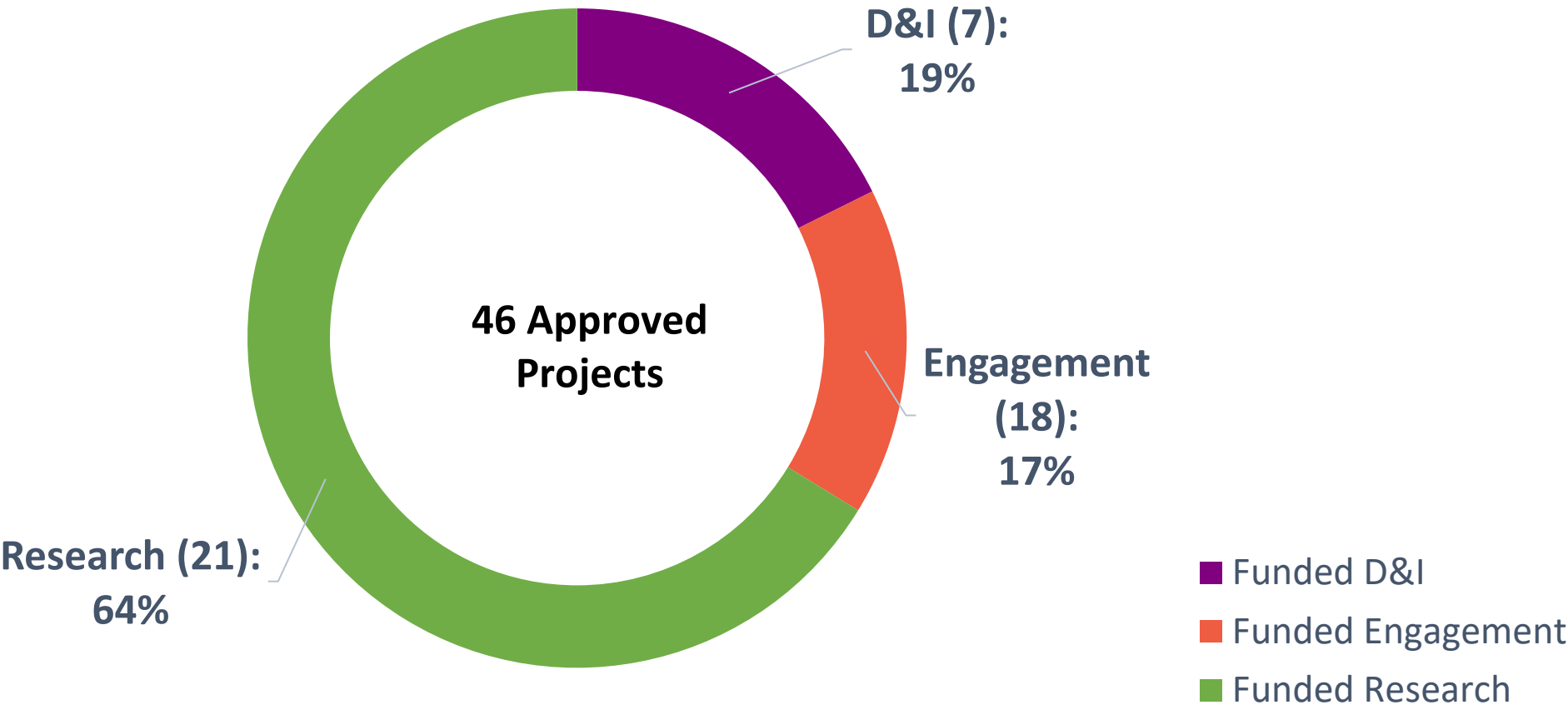
COVID-19 Enhancements – Current PCORI Funding

Health Conditions	Research Awards & Intervention Strategy								D&I Awards	Engagement Awards
	Drug	Other Clinical	Health Services	Telemedicine	Training & Education	Technology	Screening Interventions	N/A*		
Cardiovascular		■	■					■	■ (purple)	
Cancer							■			■ (orange)
Gastrointestinal		■	■							■ (orange)
Genetic Disorders										■ (orange)
Infectious Diseases									■ (purple)	
Mental/Behavioral Health				■	■					■ (orange)
Muscular & Skeletal										■ (orange)
Neurological	■								■ (purple)	
Nutritional & Metabolic Disorders					■					
Reproductive & Perinatal Health				■					■ (purple)	■ (orange)
Respiratory Diseases						■			■ (purple)	
Kidney Diseases										■ (orange)
Blood Disorders										■ (orange)
Other or Non-Specific			■						■ (purple)	■ (orange)
Rare Diseases									■ (purple)	■ (orange)

PCORI – Always Relevant
Meaningful research questions in health care delivery

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Does pre-exposure prophylaxis with HCQ reduce rates of COVID-19 among healthcare workers?

What are the most effective practices for telehealth? Can this natural experiment 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 health care disparities in COVID outcomes and how are they generalizable to disparities more broadly?

PCORI 2.0: Opportunities to Accelerate Impact on Care Delivery and Patient Health Outcomes

Engagement

- New and sustained partnerships
- Diverse stakeholder inclusion
- Bipartisan congressional support

Impact:
- *Patients, payers, systems as partners for dissemination & uptake*
- *Evidence to implementation*
- *PCORI integral in research ecosystem*

Innovation

- Patient-centered learning healthcare
- Rapid cycle of evidence to implementation
- Pragmatic approaches to ↓ disparities

Impact:
- *"Real-time" implementation of findings*
- *Uptake into guidelines & care*
- *Reduced variation in care & disparities in conditions/pops*

Operations

- Creative funding approaches
- Efficient & effective processes

Impact:
- *Nimble and adaptable methods to speed innovation to results to patients*
- *Robust pool of outstanding research (built capacity)*

PCORI 2.0: Opportunities to Accelerate Impact on Care Delivery and Patient Health Outcomes

Engagement

Impact:
- Patients, payers, systems as partners for dissemination & uptake
- Evidence to implementation
- PCORI integral in research ecosystem

Impact in Post COVID-19 Era:
- Hospitals and healthcare systems working together
- Gaps in evidence for decision-making driving research
- PCORI essential

Innovation

Impact:
- "Real-time" implementation of findings
- Uptake into guidelines & care
- Reduced variation in care & disparities in conditions/pops

Impact in Post COVID-19 Era:
- "Real-time" queries, output, and implementation
- Accelerated uptake
- Acute focus on variation in care & disparities

Operations

Impact:
- Nimble and adaptable methods to speed innovation to results to patients
- Robust pool of outstanding research (built capacity)

Impact in Post COVID-19 Era:
- Nimble and adaptable processes, research
- Clearer approach to leveraging data, technology, infrastructure
- Expanded research pool

Envisioning PCORI 2.0

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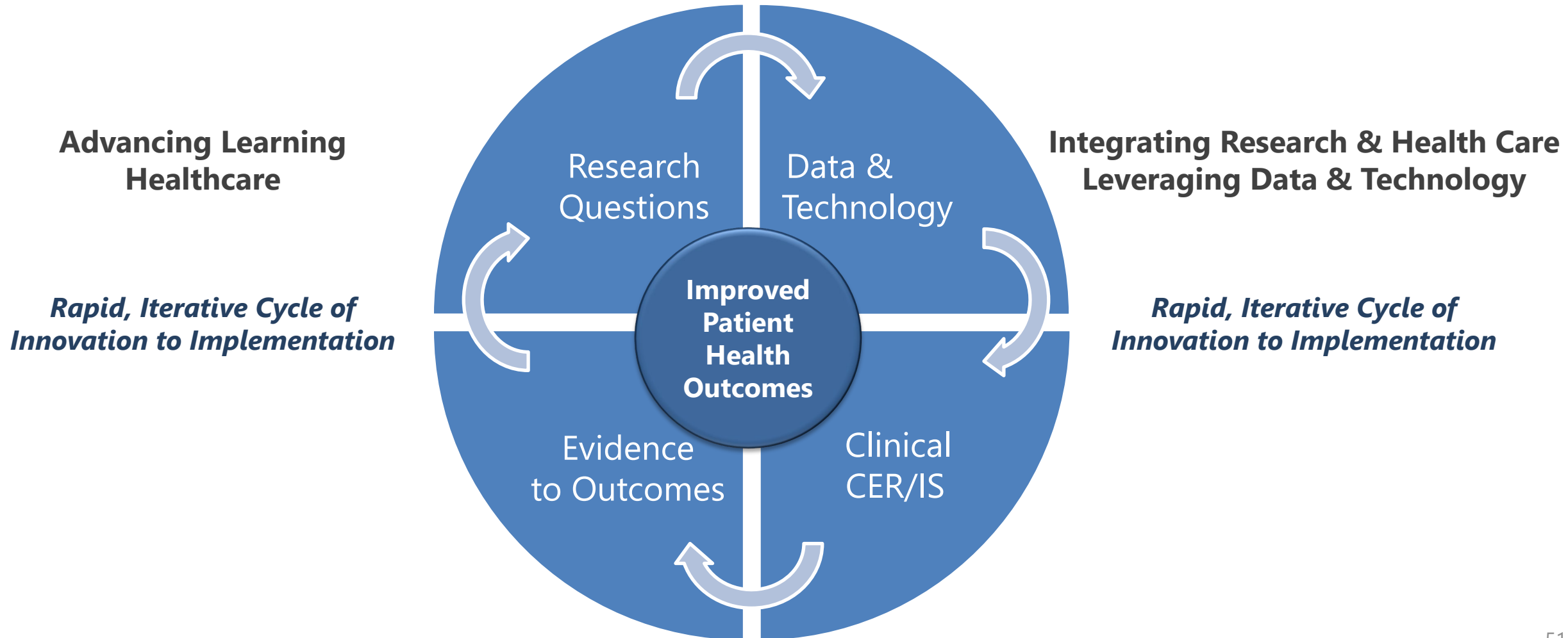| Advance Patient-Centered Learning Health Care | Reduce Health Disparities | Disseminate & Implement Scientific Findings |

Engage Stakeholders   *Crowdsource*   *Reflect, Discuss*   *Comment*

**Generate Evidence:
Patient-Centered & Relevant to Patients**

PCORI 2.0: A Virtuous Cycle of Evidence to Implementation

Advancing Learning Healthcare

Integrating Research & Health Care Leveraging Data & Technology

Rapid, Iterative Cycle of Innovation to Implementation

Rapid, Iterative Cycle of Innovation to Implementation

Research Questions

Data & Technology

Improved Patient Health Outcomes

Evidence to Outcomes

Clinical CER/IS

Navigating Our Course Together

- **My First Few Months at PCORI**
  - My Personal Journey
  - Early Onboarding
  - Passionate and Mission-Driven PCORI Staff and Board

- **Envisioning PCORI 2.0**
  - Opportunities for the Future
  - Learning in a Public Health Crisis

- **Our Course Together**
  - Year 1 Priorities

Navigating Our Course Together

First Year Focus

- Onboarding and Virtual Listening Tour

- PCORI's Response to the COVID-19 Pandemic

- National Priority Setting and Development of Research Agenda and Strategic Plan

- Other Priorities Stemming from Legislation (e.g., Maternal Mortality, Intellectual and Developmental Disabilities, Cost Outcomes)

Collaborative Strategy to Advance a Vision for PCORI 2.0

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Refreshing National Priorities, Research Agenda, and Strategic Plan

Garner diverse input to guide scientific directions
Patients, Clinicians, Researchers, Purchasers, Payers, Industry, Hospitals & Health Systems, Policy Makers, Training Institutions

Listening Tour
Board & Staff

Conduct portfolio analyses & evaluate research programs

Refine priority research areas

Develop implementation plans

Align resources with research priorities

HDDR Advisory Panel
National Priority Setting and Research Agenda

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What are the driving questions to fulfill PCORI's comparative clinical effectiveness research agenda?

How do we generate the evidence to advance the health system's ability to address health equity?

How do we advance the important work of Addressing Disparities amid the current pandemic?

What are the critical resource and workforce issues to implement these considerations for the next phase for PCORI?

Questions?

Break

1:15-1:45 PM Eastern

Webinar will resume at 1:45 PM

Agenda

1. Statutory Mandates
2. Current National Priorities
3. Looking Forward: Panelists' Perspectives on a Refresh

1.

Statutory Mandate

National Priorities

- Required under PCORI's authorizing law.

- Identification is a core duty—the first enumerated duty—of the Institute

- Intended to guide PCORI and provide transparency to the public on the Institute's investment strategy in patient-centered comparative clinical effectiveness research

Subtitle D—Patient-Centered Outcomes Research

SEC. 6301. PATIENT-CENTERED OUTCOMES RESEARCH.

(a) IN GENERAL.—Title XI of the Social Security Act (42 U.S.C. 1301 et seq.) is amended by adding at the end the following new part:

"PART D—COMPARATIVE CLINICAL EFFECTIVENESS RESEARCH

"COMPARATIVE CLINICAL EFFECTIVENESS RESEARCH

"SEC. 1181 [42 U.S.C. 1320e]. (a) DEFINITIONS.—In this section:

"(1) BOARD.—The term 'Board' means the Board of Governors established under subsection (f).

"(2) COMPARATIVE CLINICAL EFFECTIVENESS RESEARCH; RESEARCH.—

"(A) IN GENERAL.—The terms 'comparative clinical effectiveness research' and 'research' mean research evaluating and comparing health outcomes and the clinical effectiveness, risks, and benefits of 2 or more medical treatments, services, and items described in subparagraph (B).

"(B) MEDICAL TREATMENTS, SERVICES, AND ITEMS DESCRIBED.—The medical treatments, services, and items described in this subparagraph are health care interventions, protocols for treatment, care management, and delivery, procedures, medical devices, diagnostic tools, pharmaceuticals (including drugs and biologicals), integrative health practices, and any other strategies or items being used in the treatment, management, and diagnosis of, or prevention of illness or injury in, individuals.
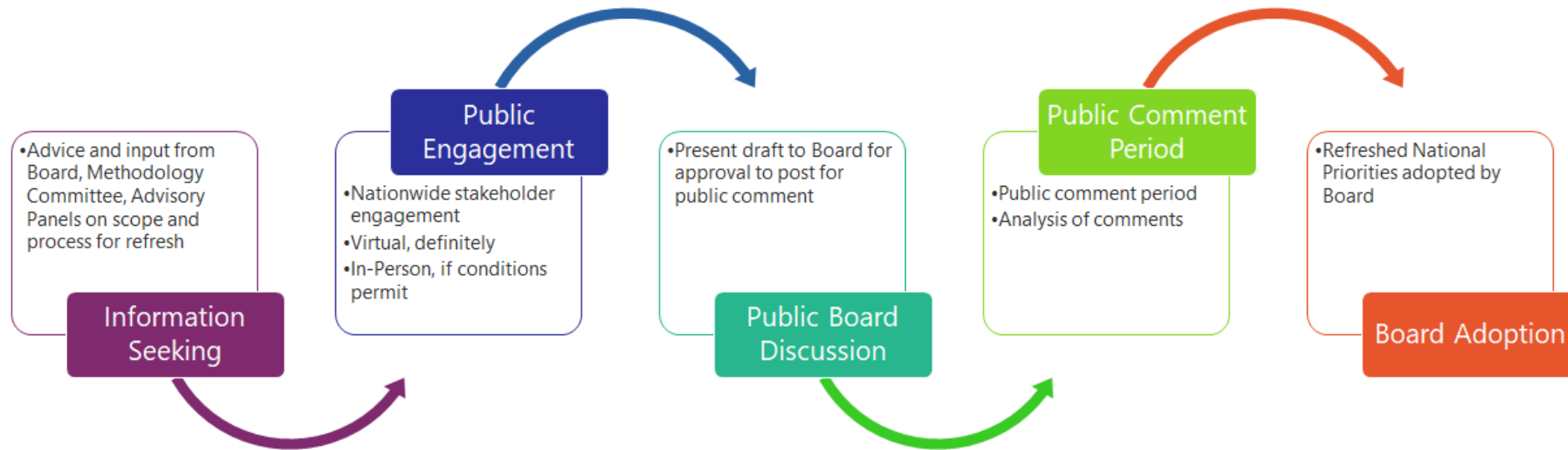
National Priorities

- The Institute shall identify national priorities for research, taking into account **factors of disease** incidence, prevalence, and burden in the United States (with emphasis on chronic conditions), **gaps in evidence** in terms of clinical outcomes, **practice variations** and **health disparities** in terms of delivery and outcomes of care, the **potential for new evidence** to improve patient health, well-being, and the quality of care, the effect on national expenditures associated with a health care treatment, strategy, or health conditions, as well as patient needs, outcomes, and preferences, the relevance to patients and clinicians in making informed health decisions...
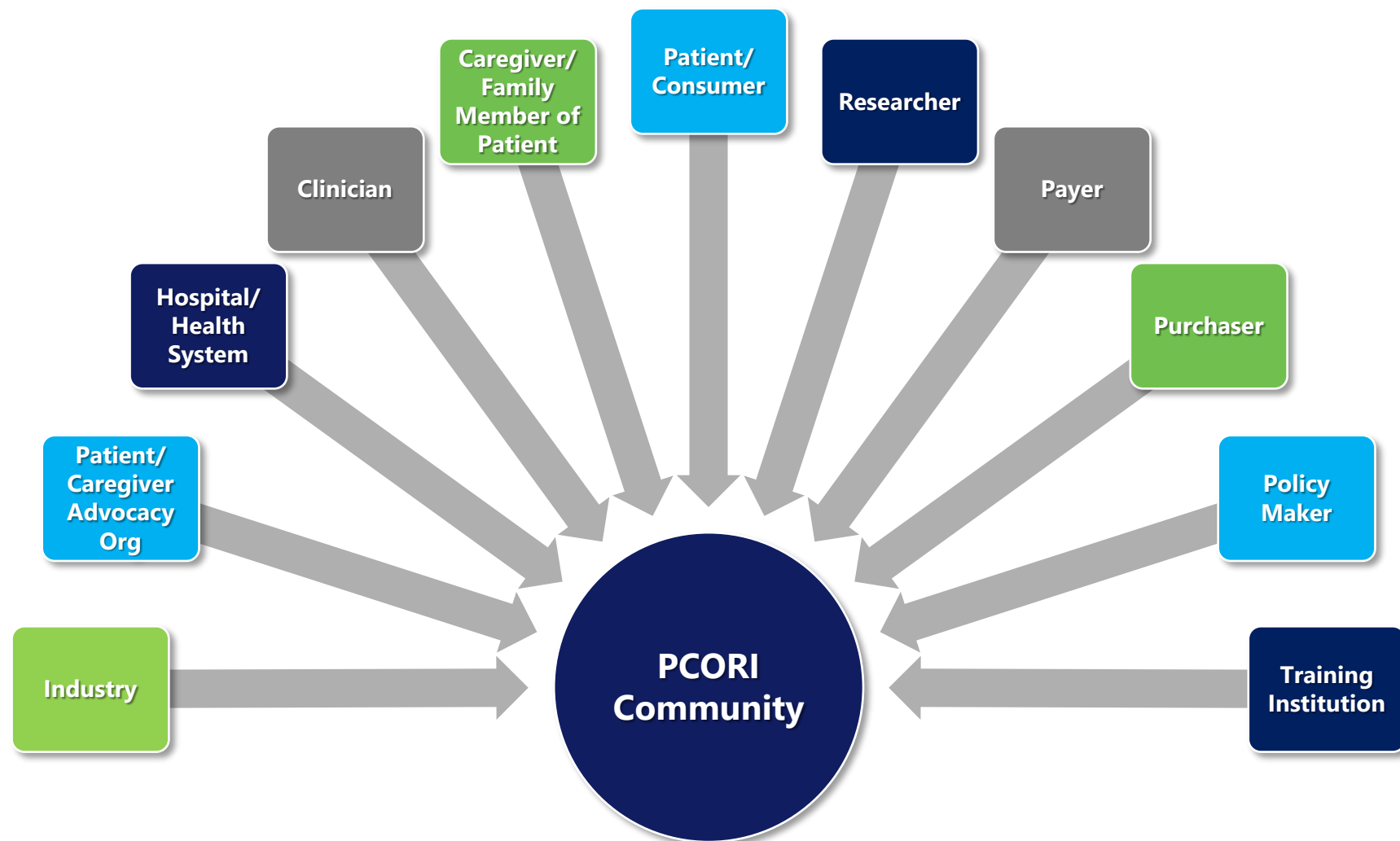
*emphasis added

Mandated Process

- Formal Public Comment is required
  - Minimum: 45 days
  - Maximum: 60 days
- Final approval/adoption rests with PCORI Board of Governors

Whom We Will Engage

- PCORI
  - Board of Governors
  - Methodology Committee
  - Advisory Panels

- Our Stakeholders across the Nation

2.

Current National Priorities

Our Current National Priorities

Our prior approach

- Broad national priorities
- Articulation of criteria to inform the *process* for establishing a research agenda

Assessment of Prevention, Diagnosis, and Treatment Options
- Comparing the effectiveness and safety of alternative prevention, diagnosis, and treatment options to see which ones work best for different people with a particular health problem.

Improving Healthcare Systems
- Comparing health system-level approaches to improving access, supporting patient self-care, innovative use of health information technology, coordinating care for complex conditions, and deploying workforce effectively.

Communication and Dissemination Research
- Comparing approaches to providing comparative effectiveness research information, empowering people to ask for and use the information, and supporting shared decision-making between patients and their providers.

Addressing Disparities
- Identifying potential differences in prevention, diagnosis or treatment effectiveness, or preferred clinical outcomes across patient populations and the healthcare required to achieve best outcomes in each population.

Accelerating Patient-Centered Outcomes Research and Methodological Research
- Improving the nation's capacity to conduct patient-centered outcomes research, by building data infrastructure, improving analytic methods, and training researchers, patients and other stakeholders to participate in this research.

3.

Looking Forward: Perspectives on a Refresh

We Want to Hear from You

- Questions and Discussion

PCORI Reauthorization Briefing

Jean Slutsky

Congressional Leaders

House Champions

Diana DeGette (D-CO)
Energy & Commerce

Don Beyer (D-VA)
Ways & Means

Senate Champions

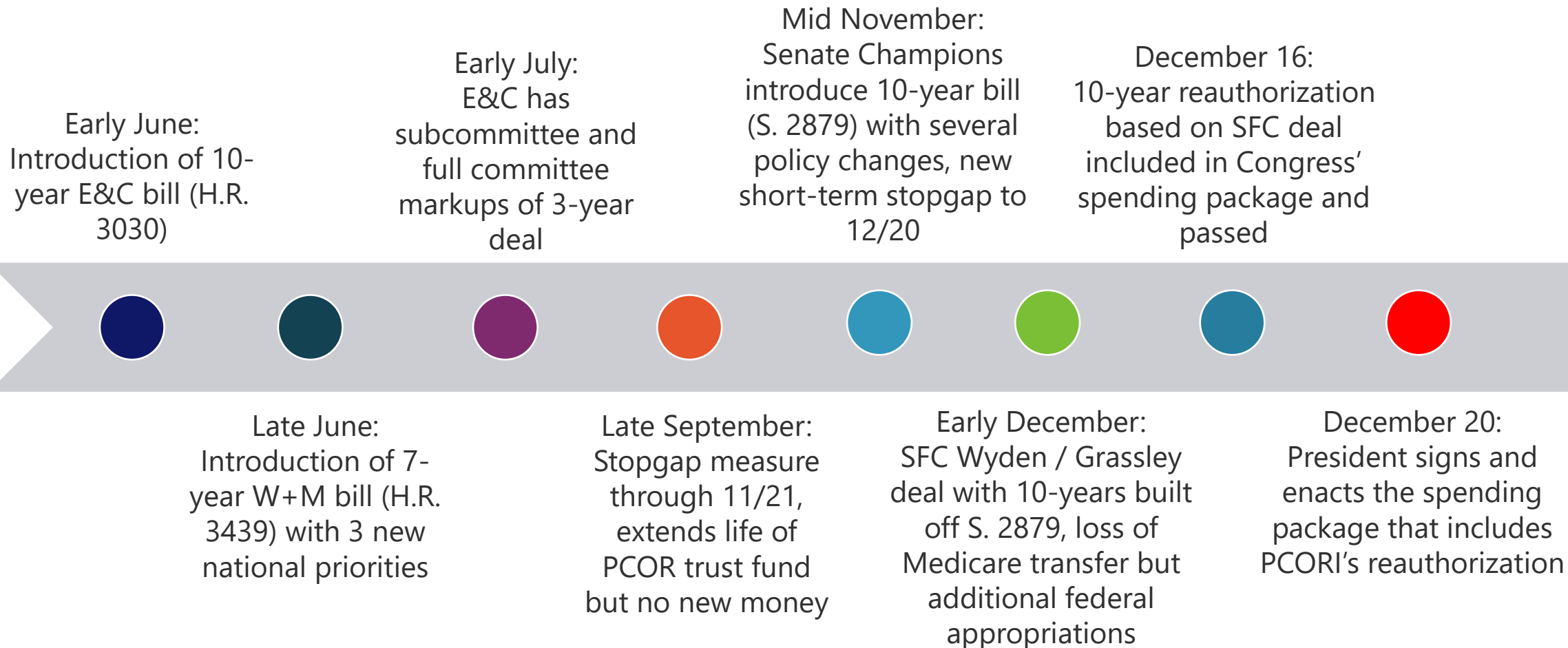Mark Warner (D-VA)

Bill Cassidy (R-LA)

Chris Van Hollen (D-MD)

Shelly Moore Capito (R-WV)

Legislative Activity

Early June:
Introduction of 10-year E&C bill (H.R. 3030)

Late June:
Introduction of 7-year W+M bill (H.R. 3439) with 3 new national priorities

Early July:
E&C has subcommittee and full committee markups of 3-year deal

Late September:
Stopgap measure through 11/21, extends life of PCOR trust fund but no new money

Mid November:
Senate Champions introduce 10-year bill (S. 2879) with several policy changes, new short-term stopgap to 12/20

Early December:
SFC Wyden / Grassley deal with 10-years built off S. 2879, loss of Medicare transfer but additional federal appropriations

December 16:
10-year reauthorization based on SFC deal included in Congress' spending package and passed

December 20:
President signs and enacts the spending package that includes PCORI's reauthorization

PCORI's Reauthorization Language

Overview of What the Final Reauthorization Language Looks Like

- Extends funding for **10 years**

- Replaces funding from the **Medicare Trust Fund** by increasing **mandatory federal appropriations** to the PCOR Trust Fund

- Adds **intellectual and developmental disabilities** and **maternal mortality** as research priorities

- Requires PCORI to **balance long- and short-term priorities** when identifying research priorities

- Permits PCORI funded studies to **collect certain economic data**, where appropriate

- Increases **private payers representatives** on the Board by up to two slots

- Shifts **Methodology Committee members** appointments to the **PCORI Board**

- Strengthens and formalizes PCORI's **dissemination and implementation** mandate

- Directs GAO to review certain **barriers** to conducting research

- GAO to **analyze PCORI's dissemination program**

Reauthorization for 10 Years

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PCORI authorized through

September 30, 2029

Funding will **remain approximately consistent** with previous **10 years**

Health Insurance Policies and Self-Insured Health Plans

Continues the per-life covered fee on private health insurances, self-insured and employer-sponsored health plans funding to the PCOR Trust Fund.

Identification of Research Priorities

Adds two new research priorities:

1. **Maternal mortality**
2. **Intellectual and developmental disabilities**

PCORI must reflect a **balance of short- and long-term priorities** and **be responsive to changes** in medical evidence and treatments

Consideration of Full Range of Outcomes Data

Where appropriate, PCORI-funded studies may capture data on the **potential burdens and economic impacts** of the utilization of medical treatments, items, and services for all stakeholders. This data includes:

- Medical out-of-pocket costs, including:
    - Health plan benefit
    - Formulary design
- Healthcare utilization

- Nonmedical costs to patients and family, including:
    - Caregiving
    - Effects on future costs of care
    - Workplace productivity
    - Absenteeism

This compromise language **does not** remove statutory prohibitions on PCORI establishing cost-per quality adjusted life year (QALY) thresholds or allow PCORI to conduct cost-effectiveness analyses

Board Composition

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Raises the number of PCORI Board members appointed by the GAO to at least 19, but not more than 21

Adds up to two more payer/purchaser representatives to the Board, for a total of up to five slots

- The Directors of the NIH and AHRQ continue to serve on the Board.
- Additionally, Board members who are appointed to fill a vacancy are eligible for a full-term reappointment following the completion of the remainder of their predecessor's term

Methodology Committee Appointments

Switches responsibility for appointing Methodology Committee members

From the GAO ➡️ **PCORI Board of Governors**

GAO Oversight
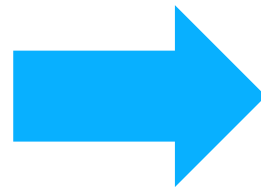
Directs the GAO to review and report to Congress about:

- PCORI's **dissemination, training, and capacity-building activities**

- **PCORI's dissemination program**

- **Any barriers researchers encounter** during studies, including challenges covering the cost of treatments, services, or items

Break

3:00-3:15 PM Eastern

Consideration of
Full Range of Outcomes Data

Jean Slutsky, Andrew Hu,
Joanna Siegel, Bill Lawrence,
Penny Mohr

Consideration of Full Range of Outcomes Data

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Consideration of Full Range of Outcomes Congressional and Stakeholder Intent

- Congressional leaders and stakeholders across the healthcare community weighed in on whether to include provisions directing PCORI to capture additional economic/cost-related data.

- The goal of this provision is to direct PCORI-funded research to better inform healthcare decisions based on the clinical effectiveness, impact on patient-reported outcomes, **and** potential economic and cost burden.

- The provision is a compromise between stakeholders to ensure PCORI-funded research capture both population-level (i.e., utilization, ROI, etc.) and patient-centric (i.e., caregiver burden, impact on medical out-of-pocket costs, etc.) data.

- This compromise language **does not** remove statutory prohibitions on PCORI establishing cost-per quality adjusted life year (QALY) thresholds or allow PCORI to conduct cost-effectiveness analyses.

Consideration of Full Range of Outcomes
Early Stakeholder Input on Implementation

Summary of the key take-aways for implementation include:

- Ensure **transparency**, notably patient engagement, throughout implementation
- Identify the **long-term goals** of capturing this data to inform implementation efforts
- Consider the **full range of treatment options** and the **investment costs** for payers and health systems to implement practice change
- Need to **develop standards** around identifying and capturing patient-centric cost data
- **Concerns around the use/misuse** of cost data that could lead to cost-effectiveness or inappropriate value assessment
- Hope to **expand beyond traditional health economic perspectives** on cost/value

Considerations of Full Range of Outcomes Tentative Implementation Proposal

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Phase 1

- Providing guidance to PIs in future PFAs on how they should interpret this policy and incorporate it into their research proposals.
- **Timeline:** Complete Phase 1 by **January 5, 2021**

Phase 2

- Establishing standards and methods to inform the field on how to capture relevant data.
- **Timeline: 12 months** from the initiation of this process

Phase 3

- Convening discussions on how this information can/should be used.
- **Timeline:** Ongoing Discussion

Thank you!

Survey

Wrap-Up and Next Steps

Nadine Barrett, PhD, MA, MS

HDDR Advisory Panel Co-Chair

Frank Wharam, MD, MPH

HDDR Advisory Panel Co-Chair

Steve Clauser, PhD, MPA

Director, Healthcare Delivery and Disparities Research Program

Panelists Completing 3-year Term

- Nadine Barrett, PhD, MA, MS

- Danielle Brooks, JD

- Cheryl Holly, EdD, MED, RN

- Mary Grace Pagaduan, MPH

- Rachel Raia, MPH

- Frank Wharam, MD, MPH

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thanks you for your service!

Meeting Adjourned