

# Clinical Effectiveness and Decision Science

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PCORI Virtual Advisory Panel Meeting

June 24, 2021 | 1:30–4 pm (ET)

June 25, 2021 | 1–4 pm (ET)

# Welcome & Introductions

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**Stanley Ip, MD**

CEDS Interim Program Director

**Kari Gali, DNP, APRN, PNP-BC**

CEDS Advisory Panel Chair

**Julie Eller, MPH Candidate**

CEDS Advisory Panel Co-chair



# Housekeeping



- **Today's webinar is open to the public and is being recorded.**
  - Pre-reading meeting materials were emailed beforehand.
    - Meeting information can be found at [www.pcori.org](http://www.pcori.org):
    - Meetings & Events → Advisory Panel Meetings → *Advisory Panel on Clinical Effectiveness and Decision Science Summer 2021 Meeting*
- No public comment period is scheduled.
- Please remember to **speak loudly and clearly into your microphone.**
- State your name and affiliation before you speak.
- Please avoid technical language in your discussion.
- As a reminder, please mute yourself when not speaking.

# Conflict of Interest Statement



Disclosures of conflicts of interest of members of this panel are publicly available on PCORI's website and are required to be updated annually. Members of this panel are also reminded to update conflict of interest disclosures if the information has changed by contacting your staff representative.

If this panel will deliberate or take action on a manner that presents a conflict of interest for you, please inform the Chair so we can discuss how to address the issue. If you have questions about conflict of interest disclosures or recusals relating to you or others, please contact your staff representative.

# CEDS Panel Leadership



- **Kari Gali, DNP, APRN, PNP-BC**  
CEDS Panel Chair
- **Julie Eller, BS, MPH Candidate**  
CEDS Panel Co-Chair
- **Holly Ramsawh, PhD**  
CEDS Senior Program Officer, Panel Manager
- **Amanda Barbeau, MPH**  
CEDS Senior Program Associate, Panel Manager

# CEDS Department Staff



**Stanley Ip, MD**  
Interim Program Director



**Kimberly Bailey, MS**  
Interim Associate Director



**Rebecca Barasky, MPH**  
Associate Director



**Anne Trontell, MD, MPH**  
Associate Director



**Bridget Gaglio, PhD, MPH**  
Senior Program Officer



**Jason Gerson, PhD**  
Senior Program Officer



**Holly Ramsawh, PhD**  
Senior Program Officer



**Yewande Akinbami, MD, MPH**  
Program Officer



**Amanda Chue, PhD**  
Program Officer



**Thuy-Vy Do, PhD**  
Program Officer



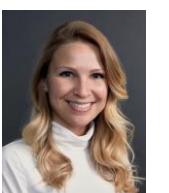
**Theresa Kim, PhD, MS**  
Program Officer



**Nora McGhee, PhD**  
Senior Program Officer



**Meghan Warren, PhD**  
Program Officer



**Amanda Barbeau, MPH**  
Senior Program Associate



**Natalia Lapinskaya, MSc**  
Program Associate



**Emily Lazowick, MPH**  
Program Associate



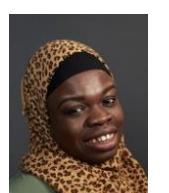
**Megan Leimkuhler**  
Program Associate



**Jillian Nowlin, MA**  
Program Associate



**Sarah Philbin, MPH**  
Senior Program Associate



**Hamidat Segunmaru, MPH**  
Program Associate



**Julia Song, MPH**  
Program Associate



**Jess Robb, MPH**  
Senior Program Associate



**Rajesh Satpathy**  
Program Associate



**Rajvi Shah**  
Program Associate



**Gbemi Alakija**  
Senior Administrative Assistant



**Arletta Prasczalek**  
Senior Administrative Assistant

# CEDS Panel Chair and Co-Chair



**Kari Gali, DNP, APRN, PNP-BC**

Pediatric Nurse Practitioner, *Cleveland Clinic*

**Representation:** Clinicians  
CEDS Advisory Panel Chair



**Julie Eller, MPH Candidate**

Director of Patient Centered Strategies, *Arthritis Foundation*

**Representation:** Patient Advocates/Patients  
CEDS Advisory Panel Co-Chair



# Day 1 Agenda, 1:30-4 pm (ET)



Time	Duration	Activity
1:30 pm	(30 min)	<b>Welcome and Introductions</b>
2 pm	(15 min)	<b>CEDS Research Area Overview</b>
2:15 pm	(90 min)	<b>Strategic Planning: National Priorities for Health and Research Agenda</b>
3:45 pm	(15 min)	<b>Closing Remarks</b>

# CEDS Advisory Panel

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Fall 2020 - Fall 2021

Members



# Patients, Caregiver Advocates, and Advocacy Organizations



Helen M. Beady, EdD, MEd



Michael Philbin, PhD

# Clinicians and Researchers



William Bennett,  
MD, MS



Karen Giuliano,  
PhD, MBA, RN



Lisa Goldman  
Rosas, PhD, MPH



Samantha Harden,  
PhD, RYT® 500



Susan Johnson,  
MBA, MS



Joey Mattingly,  
PharmD, PhD,  
MBA



Rick Rader, MD

# Payers, Industry, and Policy Makers



Adjoa Adofo  
Kyerematen,  
MS



Danielle Bargo,  
MSc



Eric Cannon,  
PharmD,  
FAMCP



David Webster,  
MD, MBA



Maureen  
White, MD, MS,  
MBA

# CEDS Program Update

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**Holly Ramsawh, PhD**  
CEDS Senior Program Officer

# Clinical Effectiveness and Decision Science



The **Clinical Effectiveness and Decision Science (CEDS)** program seeks to fill clinical information gaps by producing valid, trustworthy, and useful new evidence comparing the effectiveness of different clinical options. In situations where there already is adequate evidence, CEDS seeks approaches to raise patients' and caregivers' awareness of this information so they can make use of it in choosing the best option for them.

# CEDS Overview



Assessment of Prevention,  
Diagnosis, and Treatment Options



Improving  
Healthcare Systems



Communication &  
Dissemination  
Research



Addressing  
Disparities



Accelerating PCOR  
and Methodological  
Research

# Research Awards: Current CEDS Portfolio



PCORI HAS AWARDED OVER

**\$1 BILLION**  
TO FUND **366**

**COMPARATIVE EFFECTIVENESS STUDIES IN  
CLINICAL EFFECTIVENESS AND DECISION SCIENCE**

As of June 2021

Funding Mechanism	# of Projects
Broad	305
Pragmatic	22
Targeted	39

Note: Funded awards do not include MOU's, Pilot Projects, or IPD meta-analyses

# New Research Awards



## Recent Awards

4

Observational Type 2 Diabetes Mellitus  
Targeted PFA Awards

3

Assessment of Options Broad PFA Awards

2

Improving Methods for PCOR Broad PFA Awards

1

Conducting Rare Disease Research using PCORnet  
Targeted PFA Awards

# New Research Awards: A sampling



Study Title	PI Name	Site	PCORI Funding Announcement (PFA)
<a href="#"><u>Biologic Abatement and Capturing Kids' Outcomes &amp; Flare Frequency in Juvenile Spondyloarthritis (BACK-OFF JSpA)</u></a>	Pamela Weiss, MD, AB	Children's Hospital of Philadelphia	Cycle 1 2020 Broad: APDTO
<a href="#"><u>Methods to analyze patient-centered outcomes missing due to death in cluster-randomized trials</u></a>	Michael Harhay, PhD, MPH, MA, MS	Trustees of the University of Pennsylvania	Cycle 1 2020 Methods
<a href="#"><u>Second-line Therapies for Patients with Type 2 Diabetes and Moderate Cardiovascular Disease Risk</u></a>	Rozalina McCoy, MD, MS	Mayo Clinic	Cycle 2 2020: Diabetes Targeted PFA

# Currently in Review: Cycle 3 2020



## Awards Will Be Announced July 2021

- Broad: Improving Assessment of Options, Communication & Dissemination Research, and Improving Methods
  - Special Areas of Emphasis:
    - Improving care transitions for those with intellectual and developmental disabilities (IDD)
    - Increasing Access to and Continuity of Patient-Centered Maternal Care
- Suicide Prevention: Brief Interventions for Youth
- Phased Large Awards for Comparative Effectiveness Research (PLACER)

# Upcoming Targeted Research Opportunities



Maternal  
Morbidity and  
Mortality PFA

- Up to \$50M available
- PFA posted: May 2021
- Awards to be announced: **March 2022**

What is the comparative effectiveness of multicomponent interventions to improve early detection of, and timely care for, risk factors for postpartum complications, and for complications during the first six weeks postpartum for Black, AI/AN, Hispanic, rural, and low-SES patients?

# Upcoming Targeted Research Opportunities



Intellectual and  
Developmental  
Disabilities PFA

- Up to \$40M available
- PFA posted: May 2021
- Awards to be announced: **March 2022**

What is the comparative effectiveness of evidence-based approaches (e.g., specific pharmacologic and behavioral interventions) that address mental health conditions in individuals with intellectual and developmental disabilities?

# Upcoming Targeted Research Opportunities



## Urinary Incontinence

- Up to \$40M Funds Available
- PFA posted: May 2021
- Awards announced: **March 2022**

This Targeted PFA seeks to fund high-quality, comparative effectiveness research projects that focus on efficacious interventions for nonpregnant women with stress, urge, or mixed urinary incontinence, addressing high-priority patient- and stakeholder-guided research questions.

# Upcoming Broad Research Opportunities



2021 Cycle 2  
Broad PFA

- Assessment of options: Up to \$32M available
- Communication & dissemination research: Up to \$8M available
- Improving Methods for PCOR: Up to \$12M available
  
- PFA posted: January 2021
- Awards to be announced: **March 2022**

# Upcoming Pragmatic Clinical Studies Research Opportunities



2021 Cycle 2  
PCS PFA

- Funding available: \$90M
- PFA posted: May 2021
- Awards to be announced: **March 2022**

# Upcoming Phased Large Awards for Comparative Effectiveness Research Opportunity



2021 Cycle 3  
PLACER PFA

- Funding available: TBD
- PFA posts: September 2021
- Awards to be announced: **July 2022**

# 2021 PCORI Annual Meeting



November 17-19, 2021

Virtual Meeting

# Strategic Planning: Proposed National Priorities and Updating the Research Agenda

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**Nakela L. Cook, MD, MPH**  
PCORI Executive Director

# Purpose of Today's Conversations



## Purpose

- Update on strategic planning process
- Opportunity to engage with the proposed National Priorities for Health and gather perspectives

## Goal

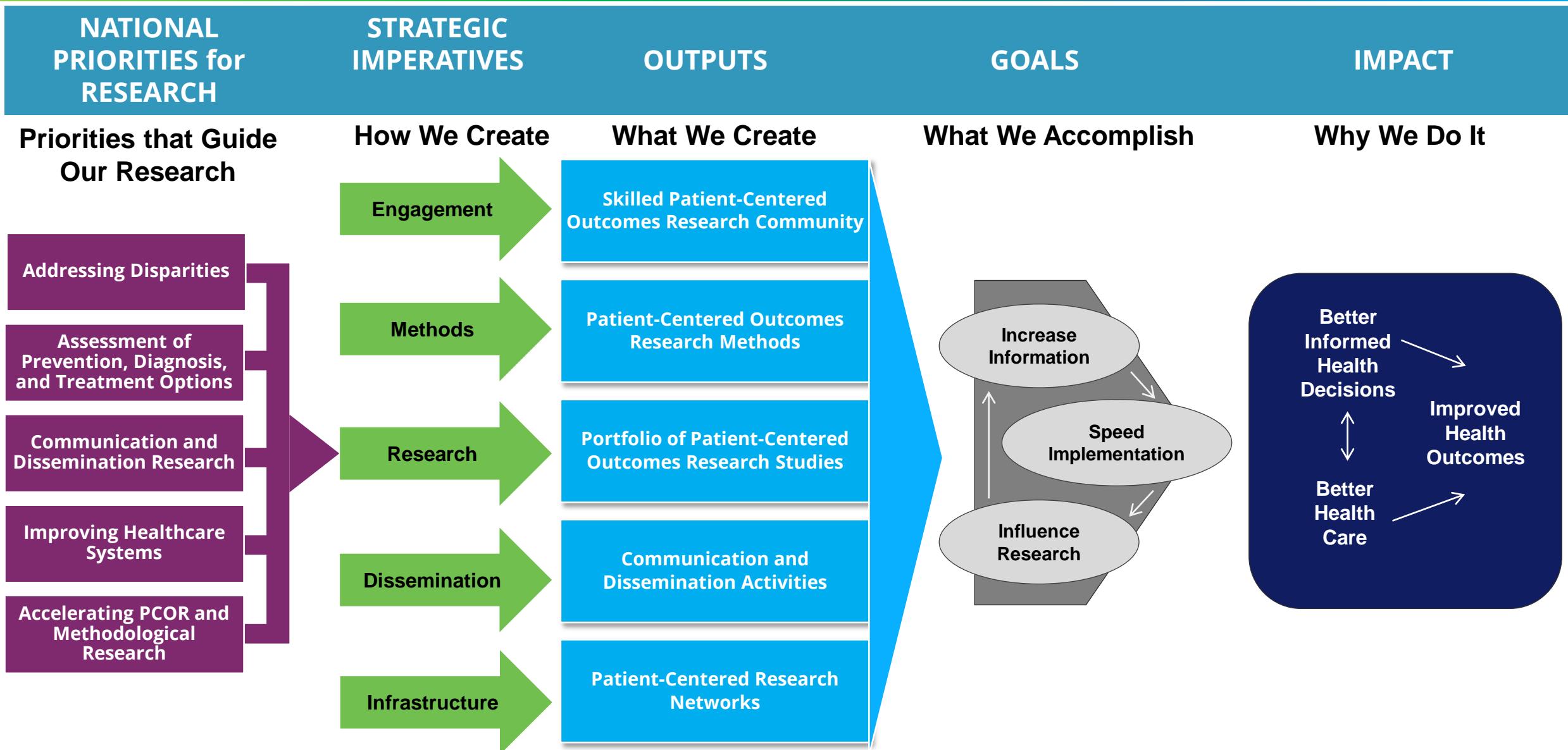
- Identify synergies among the National Priorities and consider what those may mean for the Research Agenda
- Identify needs to address through the Research Agenda

# Scope of Strategic Planning Activities

## Strategic Planning

- **National Priorities**
- Research Agenda
- PCORnet® strategic vision for PCORI's next phase
- Methodology Committee focus for PCORI's next phase
- Commitment planning and strategies to increase funding
- Scenario planning based on the changes in landscape and environment
- Priorities from reauthorizing legislation
  - Maternal morbidity and mortality
  - Intellectual and developmental disabilities
  - Full range of outcomes data

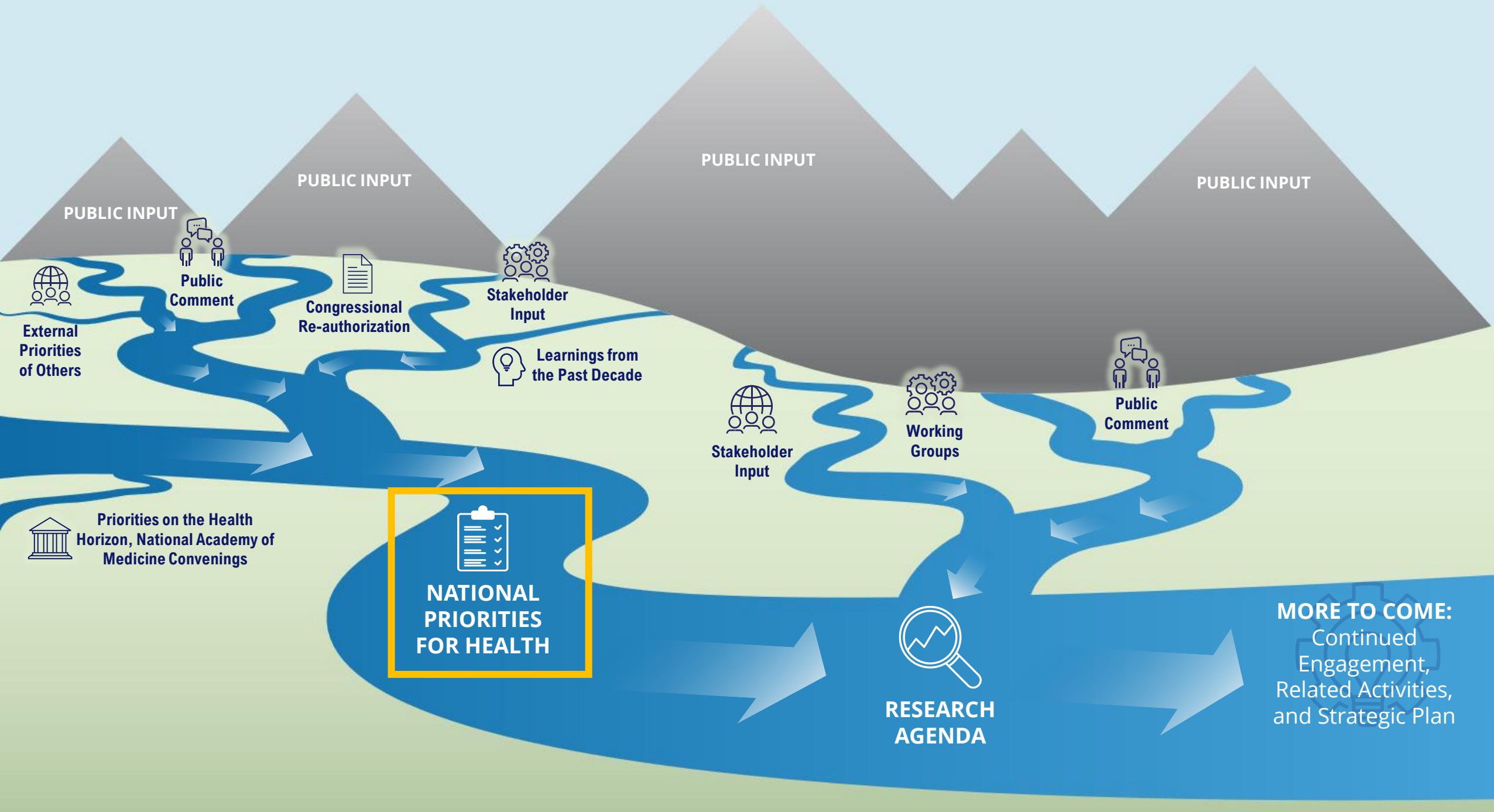
# Evolution of PCORI's Strategic Framework: Original (2013)



# Reminder about Revised Strategic Framework

## Evolving to National Priorities for Health





# Developing National Priorities for Health



- The themes below resulted from across the inputs
- At its April 2021 meeting, PCORI's Board of Governors supported developing and further shaping these themes for National Priorities for Health

**Health Equity**

**Emerging Innovations**

**Communication,  
Dissemination,  
Implementation**

**Infrastructure &  
Workforce**

**Learning Health  
System**

# Going from Themes to National Priorities

## Themes

Health Equity

Emerging Innovations

Communication,  
Dissemination,  
Implementation

Infrastructure &  
Workforce

Learning Health  
System



Achieve Health Equity

Increase Evidence for  
Existing Interventions and  
Emerging Innovations in  
Health

Advance the Science of  
Dissemination,  
Implementation, and  
Health Communication

Enhance Infrastructure to  
Accelerate Patient-  
Centered Outcomes  
Research

Accelerate Progress  
Toward an Integrated  
Learning Health System

## Proposed National Priorities for Health

# Proposed National Priorities for Health

## Creating Synergistic Opportunities for Progress



- At its June 2021 meeting, PCORI's Board of Governors approved the National Priorities for Health to be posted for public comment, which will open soon



# Proposed National Priorities for Health

## Increase Evidence for Existing Interventions and Emerging Innovations in Health



*Strengthen and expand ongoing comparative clinical effectiveness research focused on both existing interventions and emerging innovations to improve healthcare practice, health outcomes, and health equity*

### Strategies to address this priority include:

- Monitor the research landscape for potentially high-impact innovations
- Evaluate existing and emerging innovations in clinical care interventions, systems changes, healthcare delivery, technologies, public health, and social determinants of health
- Study unintended consequences, adverse events, barriers to care, burdens and economic impacts, and widened disparities in care outcomes associated with existing and emerging innovations
- Expand the scope of stakeholders engaged in PCORI's work from topic inception through implementation of the results
- Emphasize inclusion of populations who are underserved, underrepresented, and disadvantaged in CER research endeavors
- Support CER of evidence gaps in diverse populations, geographic areas, and settings to foster equitable uptake practices

# Proposed National Priorities for Health

## Enhance Infrastructure to Accelerate Patient-Centered Outcomes Research



*Enhance the infrastructure that facilitates patient-centered outcomes research to drive lasting improvements in health and transformation of both the research enterprise and care delivery*

### **Strategies to address this priority include:**

- Develop and expand the universe of engaged patients and communities and representative leadership, research workforce, and clinician partners
- Advance the accessibility and utilization of real-world data
- Build synergies and leverage current work within health systems and by stakeholders
- Integrate patient-centered outcomes research findings into learning health systems

# Proposed National Priorities for Health

Advance the Science of Dissemination, Implementation, and Health Communication



*Advance the scientific evidence for and the practice of dissemination, implementation, and health communication to accelerate the movement of comparative clinical effectiveness research results into practice*

## Strategies to address this priority include:

- Fund CER studies of delivery or implementation strategies
- Communicate research findings effectively and in ways tailored to diverse audiences
- Actively deliver information to targeted audiences to use to inform healthcare discussions and decisions
- Promote the uptake of research findings into practice to contribute to improved health care and health
- Engage stakeholders and communities in strategic partnerships across diverse settings to improve the uptake of evidence

# Proposed National Priorities for Health

## Achieve Health Equity



*Expand stakeholder engagement, research, and dissemination approaches that lead to continued progress towards achieving health equity in the United States*

### **Strategies to address this priority include:**

- Fund CER to improve health outcomes for individuals of all backgrounds
- Strengthen efforts to support inclusive and diverse stakeholder engagement
- Disseminate and implement research findings with the intention of informing broader health equity strategies
- Collaborate with health, research, advocacy, social service, educational, and other organizations to reduce health inequities
- Identify and fund novel ways to support the professional development and increase the engagement of investigators of color, investigators with disabilities, and populations who are historically underrepresented in research endeavors

# Proposed National Priorities for Health

## Accelerate Progress Toward an Integrated Learning Health System



*Foster actionable, timely, place-based, and transformative improvements in patient-centered experiences, care provision, and ultimately improved health outcomes through collaborative, multisectoral research to support a health system that serves the needs and preferences of individuals*

### Strategies to address this priority include:

- Fund multisector interventional CER focused on health outcomes and grounded in the context of specific settings, communities, and needs
- Implement research on precision and personalized medicine and whole-person health into practice
- Incorporate the full range of outcomes to influence value that encompasses diverse outcomes and perspectives among patients, families, caregivers, and providers
- Formalize partnerships to ensure an integrated learning health system that meets the needs of patients and caregivers
- Use data analytic and informatic tools to inform and enable real-time decision making

# Hearing from You

## Taking Each Priority In Turn and Considering These Questions



- Imagining five years in the future, what potential impacts could these National Priorities for Health have on CER? Are there specific areas that PCORI could make a real impact on in this timeframe?
- These National Priorities will be used to guide our Research Agenda development. What specific objectives should PCORI consider as it updates the Research Agenda?
- Where are overlapping research interests among the National Priorities and what opportunities might those create?



# Day 1 Closing Remarks

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**Kari Gali**  
CEDS Chair

**Julie Eller**  
CEDS Co-Chair



# Clinical Effectiveness and Decision Science

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June 25, 2021 | 1-4 pm (ET)

# Welcome, Recap

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**Kari Gali**  
CEDS Chair

**Julie Eller**  
CEDS Co-Chair



# Day 2 Agenda, 1- 4 pm (ET)



Time	Duration	Activity
1 pm	(5 min)	<b>Welcome and Recap of Day 1</b>
1:05 pm	(60 min)	<b>COVID Funding Initiatives + Discussion</b>
2:05 pm	(10 min)	<b>Break</b>
2:15 pm	(60 min)	<b>IDD Funding Initiatives + Discussion</b>
3:15 pm	(5 min)	<b>Break</b>
3:20 pm	(30 min)	<b>Update on MM + Q&amp;A</b>
3:50 pm	(10 min)	<b>Closing Remarks</b>

# COVID-19

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**Jason Gerson, PhD**  
CEDS Senior Program Officer



# Overview – PCORI's Response to the COVID-19 Pandemic

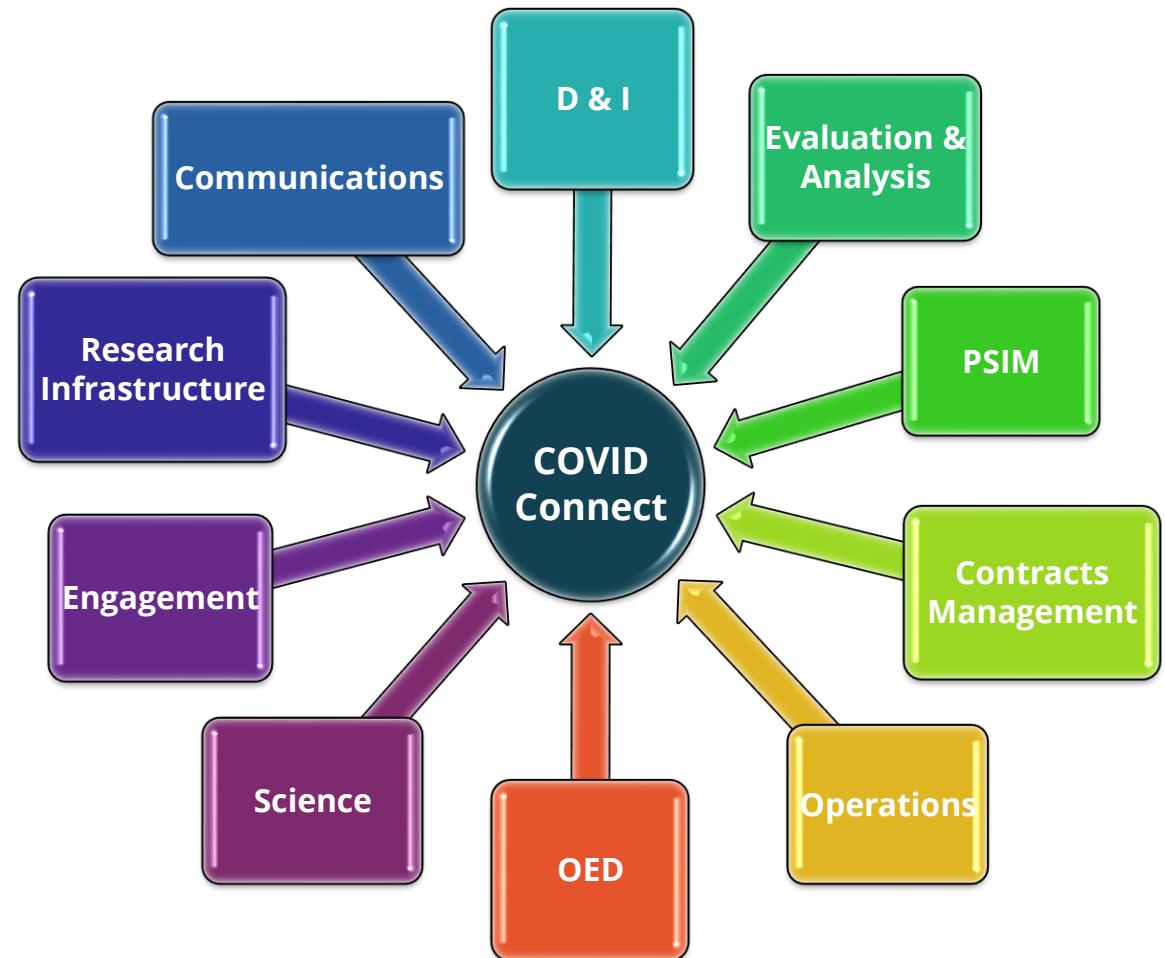


- COVID Connect
- Primary focus on COVID-related research efforts
- Brief summary of COVID-related PCORnet and engagement efforts
- Discussion

# COVID Connect: Coordinating PCORI's COVID Response



- COVID Connect is a **cross-departmental** matrixed team with representation from all of PCORI.
- Membership **evolves** based on various stages of work and need of the organization.
- Membership is voluntary and members can engage in **various levels of involvement**:
  - Full work group
  - Core subgroups
  - Ad-hoc/liaison members



# PCORI's COVID-19 Portfolio:

116 Enhancements, 9 Targeted Research Studies, and  
25 Special Cycle Engagement Awards



**116 Enhancements Awarded, \$33.5 million**

**53**

**Engagement Award**  
Enhancements  
*\$6.8 million*

**13**

**D&I**  
Enhancements  
*\$5.7 million*

**41**

**Research**  
Enhancements  
*\$18.1 million*

**8**

**Methods**  
Enhancements  
*\$2.3 million*

**1**

**PCORnet**  
Enhancement  
*\$526,020*

**34 New Awards in Research & Engagement, \$33.5 million**

**25**

**Engagement Award**  
Special Cycle  
*\$3.7 million*

**9**

**Targeted Research**  
Studies  
(COVID Targeted  
PFA)  
*\$29.8 million*

Enhancements \$ from Enhancement Tracker  
CER tPFA \$ from application amount  
Engagement Special Cycle \$ provided by EA team

# Research

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Enhancements  
COVID-19 Targeted PFAs  
Special Areas of Emphasis



# COVID Enhancements to Existing Research Projects



- Support enhancements to existing research awards that could be initiated quickly to influence the outcome of the pandemic and that have some relationship to the original award, using existing teams that are currently funded by PCORI
- Awarded on rolling basis
- \$500,000 total costs limit
- May not increase project timeline by more than 12 months
- Primary outcome assessments collected within 2 to 12 months of study initiation

# COVID Enhancements to Existing Research Projects

PCORI funded **Fifty Enhancements to Research Awards** totaling \$21.7 million



## Focus of 41 CER enhancements

13  
enhancements  
about COVID-19  
as a condition

28 enhancements  
about providing care  
during a pandemic

## Themes from 8 Methods Enhancements

2 Developing clinical prediction models

2 Informing COVID-19 care

Other themes: Data visualization, machine learning

*Note: Studies may include more than one theme*



## One PCORnet Enhancement

"optimize and rigorously validate key COVID-19 data elements related to the treatment and outcomes associated with **COVID-19 coagulopathy**"

Condition Categories	# of studies*
	Mental & Behavioral Health
	Nutritional & Metabolic
	Neurological
	Cancer
	Cardiovascular

## Key statistics about CER Enhancements:

Priority Populations	# of studies*
	Black, Indigenous, and People of Color
	Low Income
	Women
	Older Adults

## Telehealth



19 enhancements include telehealth components

# COVID-19 Targeted PFA



- PFA developed and posted on accelerated timeline in response to the urgency of the pandemic; accelerated merit review and programmatic review to ensure timely decision making
- Priority areas:
  - Adaptations to healthcare delivery
  - Impact of COVID-19 on disproportionately affected populations
  - Impact of COVID-19 on healthcare workforce well-being, management, and training
- PFA posted in May 2020; 9 awards announced in August 2020
- Studies up to 2 years in duration; actionable findings within first 12 months
- Small studies: Up to \$2,500,000; Large studies: Up to \$5,000,000

# COVID Targeted PFA Research Projects



PCORI funded **9 Targeted COVID-19 Research Awards** totaling \$29.8 million



## Focus of Awards

5 awards focus on COVID-19 as a condition

4 awards focus on ways to provide care during a pandemic



## Themes

3 targeted awards are relevant to nursing homes or other **congregate living settings**

Primary Condition	# of studies*
	COVID-19
	Mental/Behavioral Health
	Non-Disease Specific

\*Studies may include more than one condition

## Key statistics about targeted studies:

Priority Populations	# of studies*
	Low Income
	Black, Indigenous, and People of Color
	People with MCC

## Telehealth



5 targeted awards include telehealth components

# Evaluating the Comparative Effectiveness of Telemedicine in Primary Care: Learning from the COVID-19 Pandemic



## What This Study Does

- Deeply characterizes features of new or expanded telemedicine programs in primary care implemented during the COVID-19 pandemic
- Compares the effectiveness of three primary care practice delivery models under COVID-19: primarily synchronous telemedicine, telemedicine supplemented with in-person visits, and primarily in-person visits

## Design

- Observational cohort with strong qualitative component
  - Sample size: 205,000
  - Clusters: 110 primary care practices

## Key Outcomes

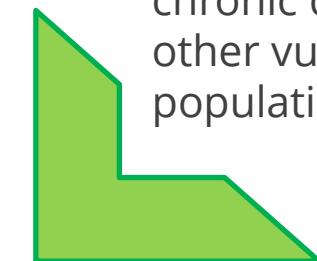
- **Primary:** Avoidable ED visits, unplanned hospitalizations, continuity of care, days at home
- **Secondary:** Patient satisfaction, communication quality, accessibility/convenience of care

## Population & Setting

- Adult patients with one or more of five chronic conditions (asthma, COPD, CHF, diabetes, hypertension) receiving care at primary care practices

## Why It Matters

Could provide much needed information on how to effectively implement telemedicine in primary care, particularly for patients with chronic disease, and for other vulnerable populations.



*Jessica Ancker, PhD FACMI  
Joan & Sanford I. Weill  
Medical College of Cornell  
University  
COVID-19 Targeted PFA*



# Increasing Vaccine Confidence among Long-Term Care Workers – Targeted PFA



- Uses an expedited mechanism; will follow more rapid review and award timeline
- PFA posted April 13, 2021
- Up to three years
- Up to \$5,000,000
- What interventions are effective in increasing COVID-19 vaccine confidence and uptake among long-term care workers?

# Broad PFA Special Area of Emphasis: Post-Acute COVID-19



## Cycle 1 2021

- 16 LOIs addressed COVID special areas of emphasis
- Management and survivorship of post-acute COVID-19
- Impact of COVID-19 on disproportionately affected populations
- Impact of COVID-19-related social isolation and loneliness on health outcomes

## Cycle 2 2021

- 6 LOIs addressed COVID special areas of emphasis
- Treatment and survivorship of post-acute COVID-19
- Health system and healthcare delivery management of post-acute COVID-19
- Strategies to improve outcomes of COVID-19 for disproportionately affected populations
- Impact of COVID-19-related social isolation and loneliness on health outcomes

# Other COVID-19 Activities

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# PCORnet - HERO Program



## HERO Healthcare Worker Registry

- >26,000 HCWs enrolled as of March 2021
- Addition of family members
- HERO Together: Pfizer-funded study on long-term vaccine side effects

## Hydroxychloroquine Trial

- Completed Feb 2021
- 1,363 enrolled
- Manuscript submission in early summer



The screenshot shows the HERO Registry homepage. The logo 'HERO' is at the top left, with the tagline 'Healthcare Worker Exposure Response & Outcomes' below it. A navigation bar with links to 'HOME', 'HERO-TOGETHER', 'HERO-HCQ TRIAL', 'VIDEO', and 'NEWS' is at the top right. The main content area features a dark blue background with white text. It says 'The HERO Registry Community is fighting COVID-19 together.' with a 'JOIN the HERO Registry' button. Below that, it says '26,568 healthcare workers are enrolled'. To the right is a grid of nine small images showing various healthcare workers in different settings.



Help us spread the word on social media by tagging  
@heroesresearch and using the hashtag #HERORegistry

HERO

# Engagement



- 53 enhancements to Engagement Awards
- 25 Engagement Awards in response to special cycle Targeted COVID-19 PFA
  - A special Targeted COVID-19 PFA is currently open
- 13 enhancements to Dissemination and Implementation Awards
- COVID-19 Supplement to the Health Care Horizon Scanning System

# Discussion Questions



- **Are there other activities we should consider?**
- **Are there specific topics that you would consider a priority for research?**

# Contact Information



Jason Gerson



Questions?



jgerson@pcori.org



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



@pcori



/PCORInstitute



PCORI



/pcori

# BREAK

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# Intellectual and Developmental Disabilities

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**Holly Ramsawh, PhD**  
CEDS Senior Program Officer



# Intellectual and Developmental Disabilities (IDD) Background



**2010** - PCORI original authorizing legislation created a process for setting national priority agenda but did not specify specific areas

**2019** – Congress reauthorizes PCORI; amending legislation specifies two new national priority areas

- **Maternal mortality**
- **Individuals with intellectual and developmental disabilities**

**This means IDD is set as a national priority area by law, thus a long-term priority area of investment → Ongoing opportunities for engagement**

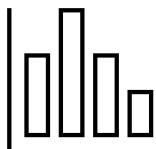
- **In months and years ahead we will be hosting a variety of opportunities to engage with us on this topic**

# IDD Background: Definition and Prevalence



## Intellectual and Developmental Disabilities:

- **Developmental disabilities** are chronic disabilities that originate at birth or in the developmental period and cause impairment in physical, learning, language, and/or behavioral areas.
- **Intellectual disabilities**, which fall under the umbrella term of developmental disabilities, involve limitations to cognitive function (i.e., reasoning, learning, problem solving) and adaptive behavior.

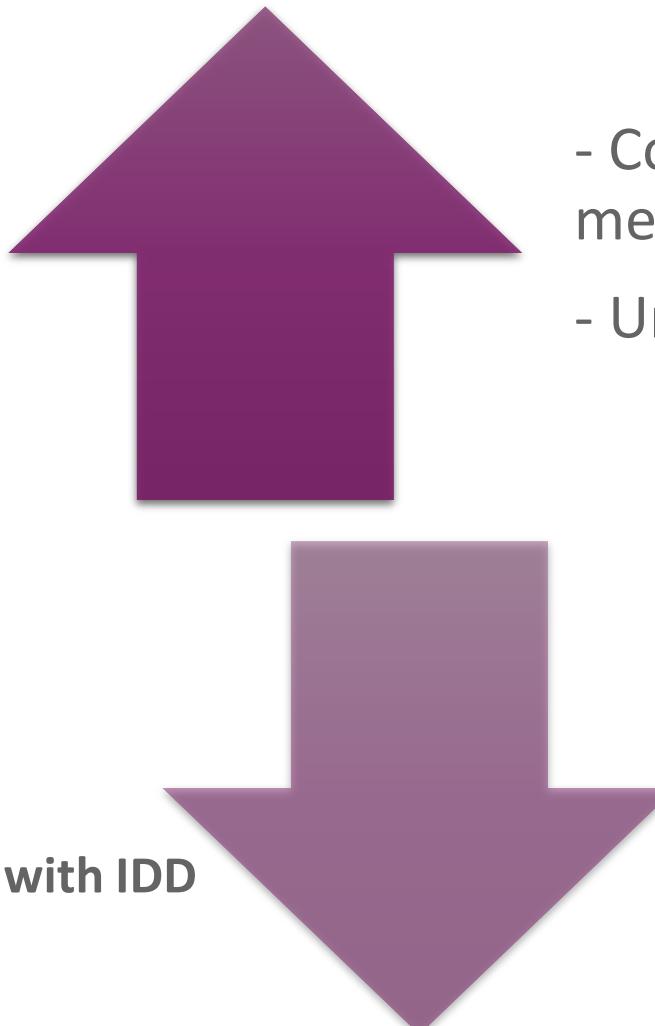


Data from the United States estimate that 17.8% of children and adolescents and 0.5 - 1.5% of adults have an IDD diagnosis, impacting 7 million to 8 million Americans.

# IDD Background: Disparities



Compared to the general population:



- Co-occurring physical and mental health conditions
- Unmet healthcare needs
- Quality of life
- Participation in school, work, play

Greater disparities exist among those with IDD for communities that are historically underserved.

# IDD Background: Co-occurring Mental Health Conditions



- Mental health conditions are **more common**; prevalence estimates of **37%-55%**
- High degree of **heterogeneity of symptoms**, but commonly occurring conditions include **mood** and **anxiety disorders**
- Complicated by **fragmentation of care delivery**
- This presents a unique set of needs/challenges across the lifespan, such as:
  - **Lower quality of care and worse health outcomes than the general population**
  - **Difficulty accessing services**
  - **Greater family burden**
  - **Poorer quality of life**
  - **Negative impact on education engagement and employment**
  - **Poorer achievement in adult life**

# Gaps in IDD and Mental Health Evidence



**Few treatment studies in this subpopulation of IDD exist due to highly restrictive inclusion criteria and of the studies that do exist, there are significant methodological limitations.**

## Gaps to be filled:

- Evidence-based mental health treatments for IDD
  - E.g., studies that evaluate the effectiveness of psychotherapeutic and cognitive behavioral therapy (CBT) modalities in children with IDD
- Larger more rigorous clinical trials
- Adaptations to existing psychosocial interventions
- Accessible treatment for rural/underserved populations
- Evidence-based research across the spectrum of IDD impairment from mild to severe and across the lifespan

# IDD Funding at PCORI



- **Special Area of Emphasis in Broad PFA:**
  - Cycles: 3 - 2020, 1 – 2021, and 2 - 2021
  - Focus: **Improving Care for Individuals with Intellectual and/or Developmental Disabilities Growing into Adulthood**
- **Research priority in PLACER and Engagement Award PFAs**
- **Targeted PFA**
  - Cycle: 2 – 2021
  - Focus: **Interventions Targeting Mental Health Conditions in Individuals with IDD**

# Targeted PFA Overview



- **Priority Research Question:** What is the comparative effectiveness of evidence-based approaches (e.g., specific pharmacologic and behavioral interventions) that address mental health conditions in individuals with IDD?
  - Interested in pharmacological, behavioral, other nonpharmacological or combination interventions administered via appropriate delivery modalities (e.g., telehealth, family-based, collaborative care, group, or individual).

**Total Direct Costs:**  
Up to \$3M

**Maximum Project Period:**  
3 years

- PCORI has allocated a total of up to **\$40 million** for this Targeted PFA

# PICOTS



<b>Population</b>	Pediatric, transitional age, and adult individuals with mild to moderate IDD-related impairment, as well as those with more severe impairment due to IDDs that have co-occurring mental health conditions
<b>Intervention/Comparators</b>	Pharmacological, behavioral, other nonpharmacological, or combination interventions administered via appropriate delivery modalities (e.g., telehealth, family-based, collaborative care, group or individual)
<b>Outcomes</b>	Symptom severity, functional impairment, quality of life, adverse events, health resource utilization, or relevant caregiver outcomes, as appropriate
<b>Timing</b>	Studies up to 3 years; Up to 12-month follow-up
<b>Setting</b>	Primary and/or specialty clinics, inpatient, outpatient/community, residential, or home-based settings may be appropriate depending on the population and proposed interventions

# IDD and Co-occurring Mental Health Conditions Targeted PFA Timeline



## PFA Posted on May 4th

Letter of Intent Deadline	<b>June 1, 2021</b>
Applicants Notified of LOI Acceptance/Denial	<b>End of June 2021</b>
Application Deadline	<b>August 31, 2021</b>
Merit Review	<b>December 2021</b>
Earliest Project Start Date	<b>August 2022</b>

# Discussion Questions

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Intellectual and Developmental Disabilities



# Discussion Question 1



- **Which top three topics would you advise PCORI prioritize CEDS funds for?**
  - Comparison of early interventions for IDD
  - Comparison of strategies to treat co-occurring health conditions in IDD (e.g., gastrointestinal disorders, seizures, dental/oral health, sensory impairments)
  - Comparison of parent-/caregiver-mediated interventions for IDD
  - Comparison of caregiver support strategies to improve resilience
  - Comparison of nonpharmacological approaches for reducing self-injurious behavior in ASD
  - Assess comparative benefits and harms and effectiveness by age of medication used in combination for children and adolescents with ADHD

# Discussion Questions 2 and 3



- **What other specific IDD topics are important to patients, caregivers, clinicians, patients, advocates, policy makers, and/or other stakeholders? (Please speak to the stakeholder group you represent)**
- **Where do you see additional CEDS CER gaps more broadly in the IDD space that can be addressed by PCORI funding?**

# BREAK

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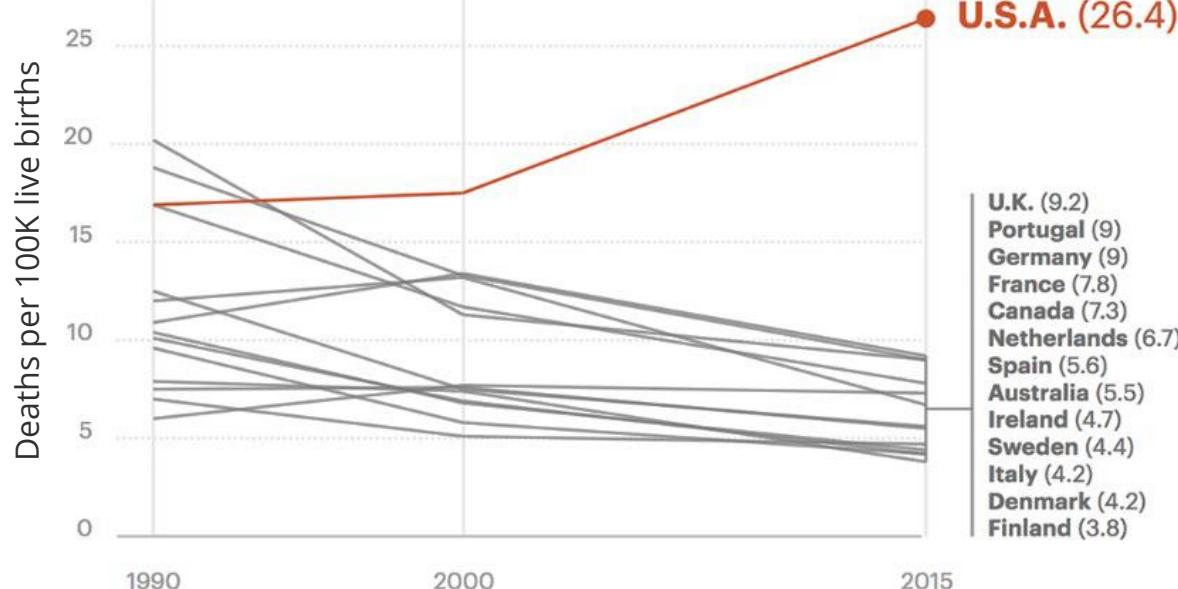
# Maternal Mortality

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**Kim Bailey, MS**  
CEDS Associate Director



# Maternal Mortality US Rates and Disparities



GBD 2015 Maternal Mortality Collaborators Agrawal P. 2015. Bulletin of the World Health Org. 93: 135.  
2016. Lancet 388: 1775-1812.

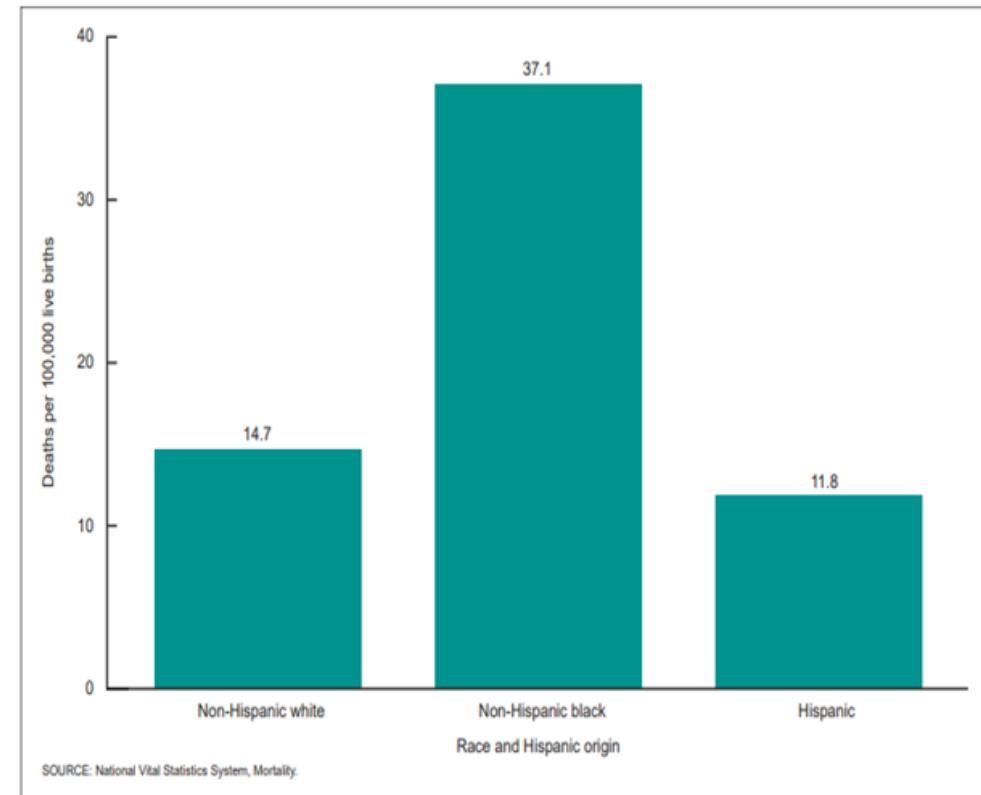
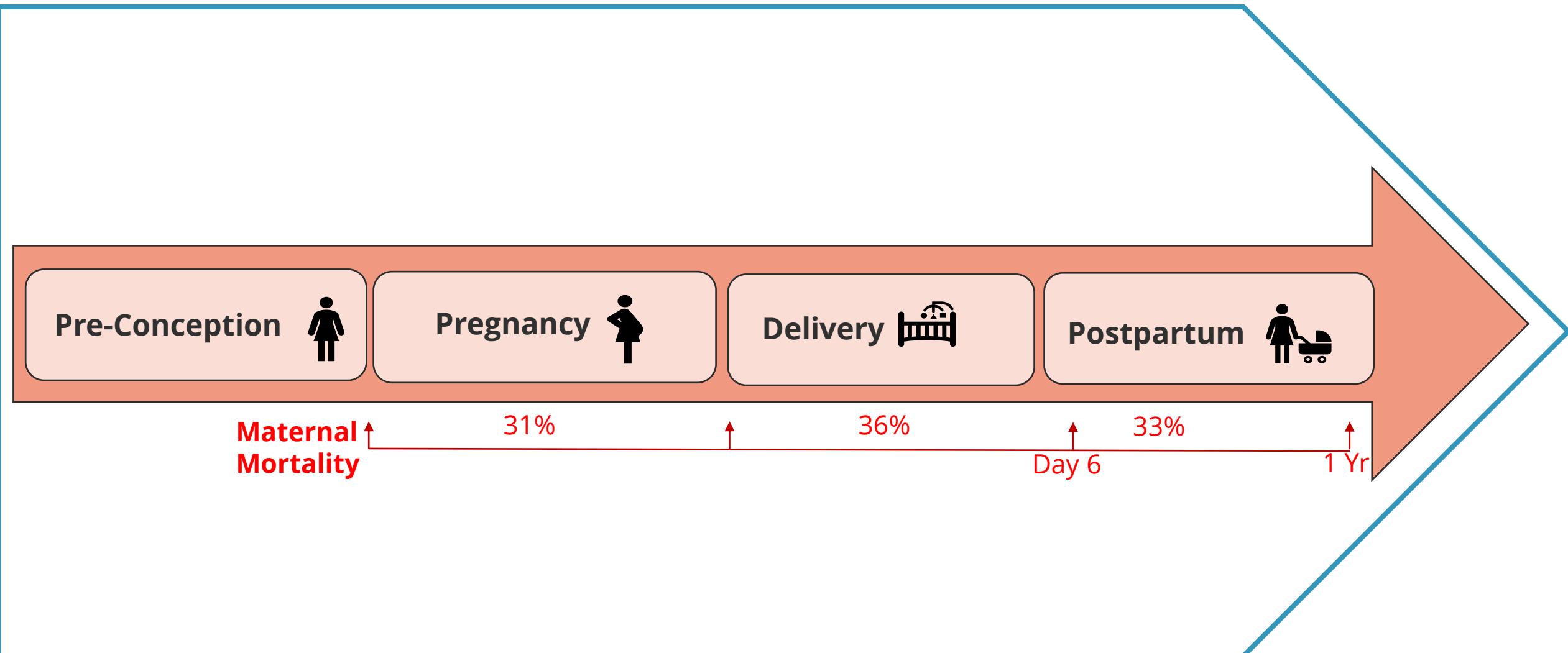
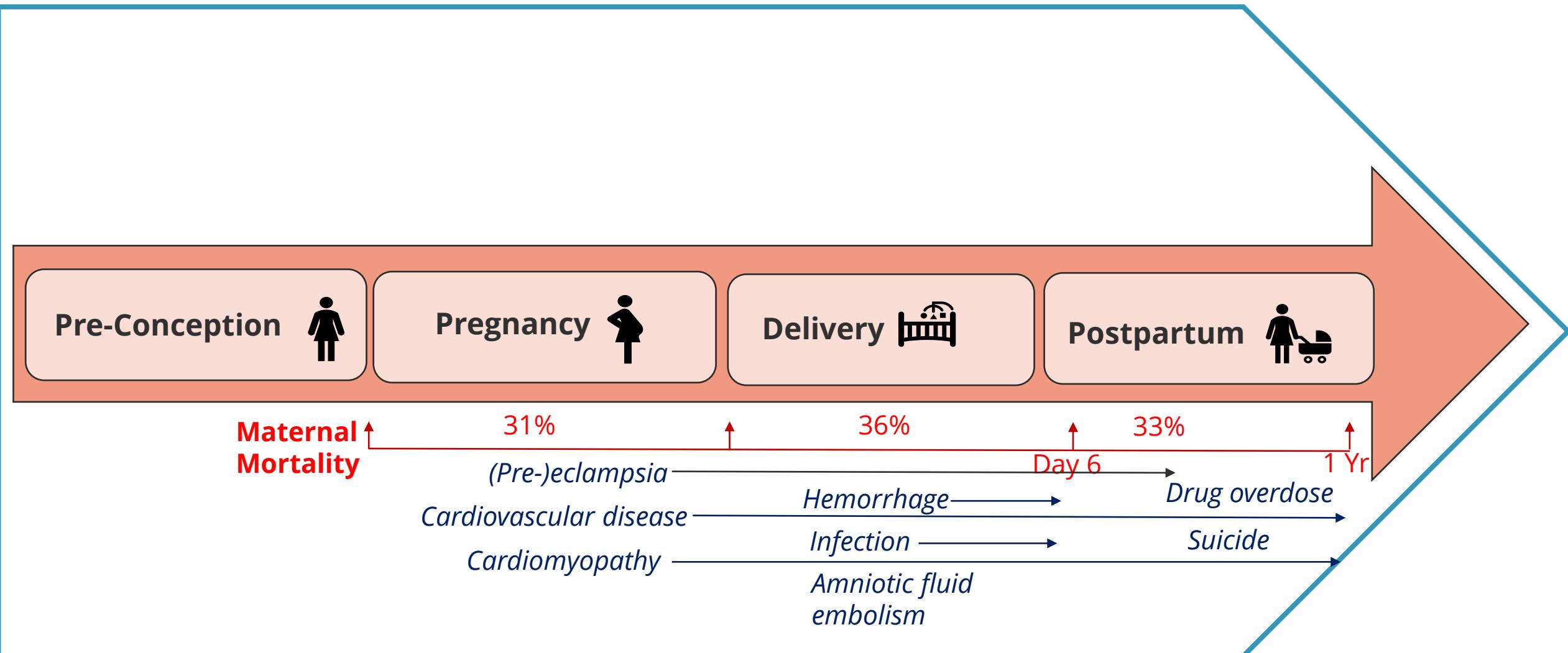


Figure 2. Maternal mortality rates, by single race and Hispanic origin: United States, 2018

# Maternal Mortality



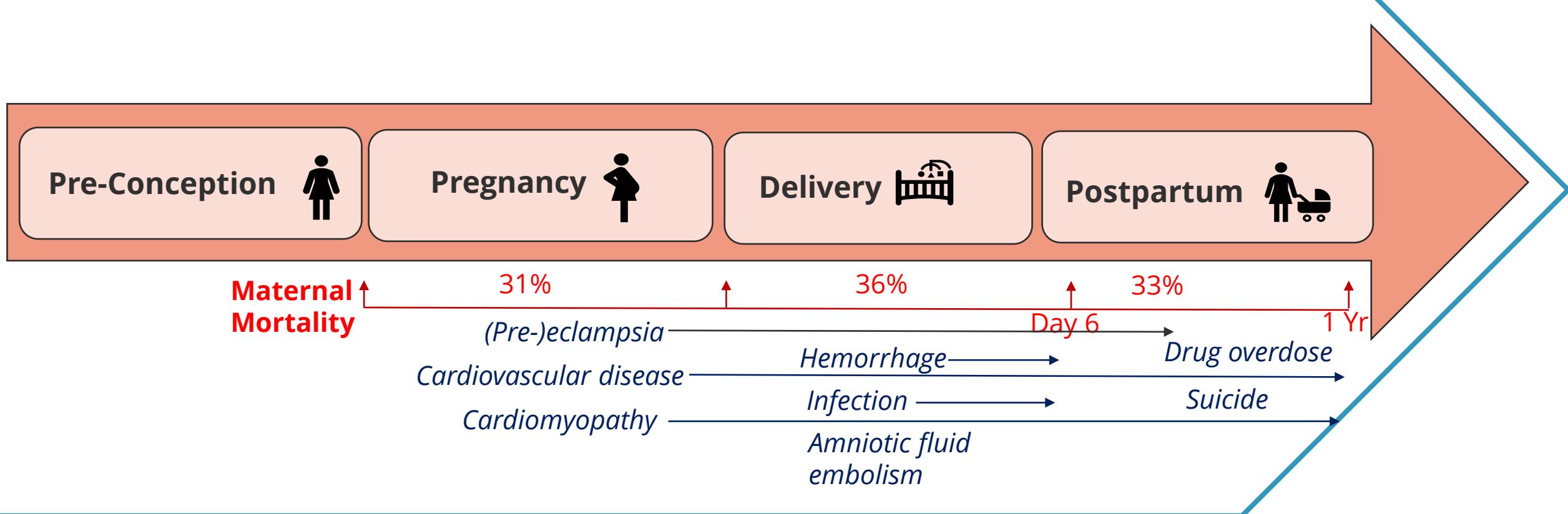
# Maternal Mortality



# Maternal Mortality



~60% of deaths preventable



# Factors Driving Maternal Mortality



## Risk Factors

- Diabetes
- BMI
- Primipara
- Smoking
- Hypertension
- Age
- Brief intrapartum period

## Social Determinants of Health

- Structural racism
- Economic stability
- Neighborhood & physical environment
- Education
- Food security
- Community & social context
- Healthcare system

# Effects of Disparities on Care and Care Experience



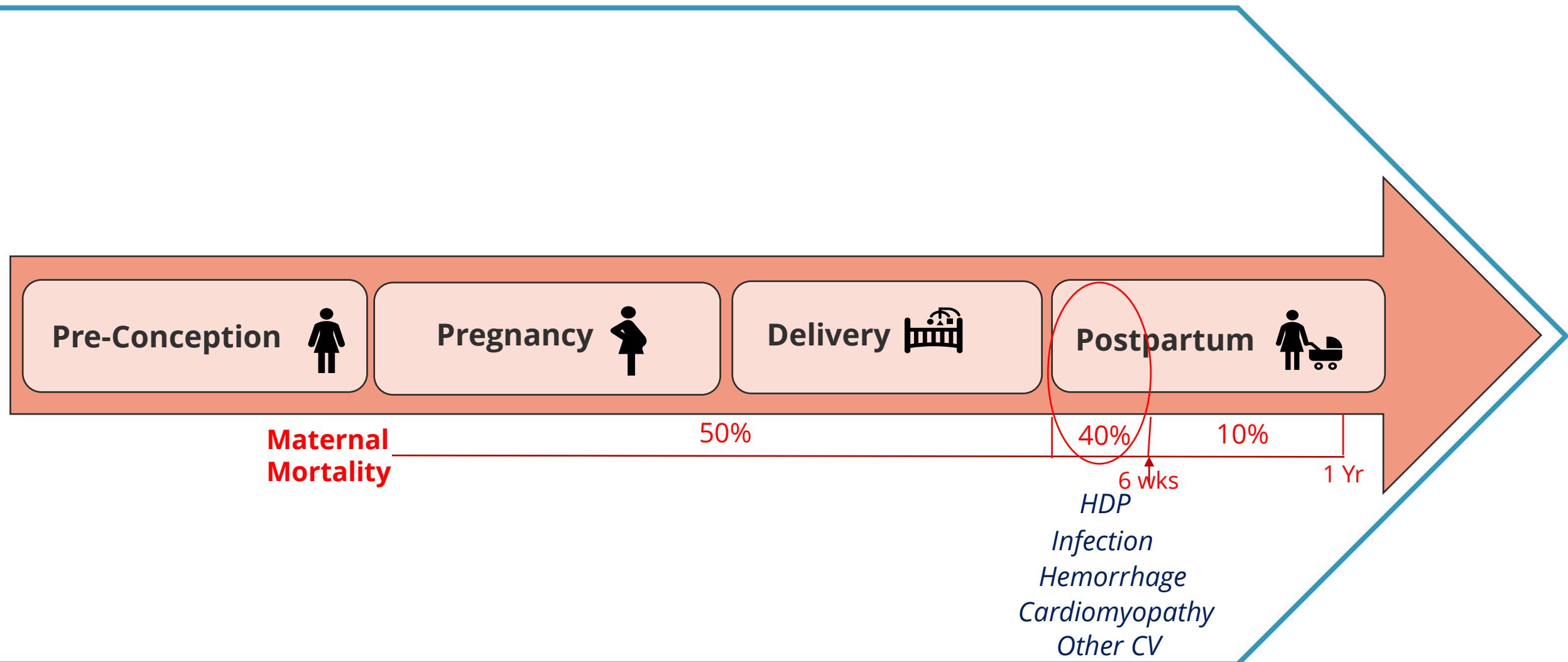
- Reduced access to care
- Delayed or missed prenatal care
- Missed or dismissed warning signs
- Negative care experience
- Reduced engagement with care

# Special Area of Emphasis: Increasing Access & Continuity of Patient-Centered Care



- Disparities are exacerbated by limited access to care and reduced engagement in care
  - Structural barriers: Distance, time, transportation, social supports, siloed care
  - Lack of patient trust
    - Clinician/staff/institution racism, cultural insensitivity, communication challenges
- **Special Area of Emphasis: Cycle 3 2020 Broad PFA onward**
  - Multilevel, culturally adapted interventions that address barriers in access to and engagement in patient-centered maternal care with a focus on:
    - Maternal care coordination
    - Education or training for healthcare providers or patients
    - Add-on or wrap-around services
  - \$30M allocation of funds
    - Studies of up to 5 years in duration and \$10M in direct costs

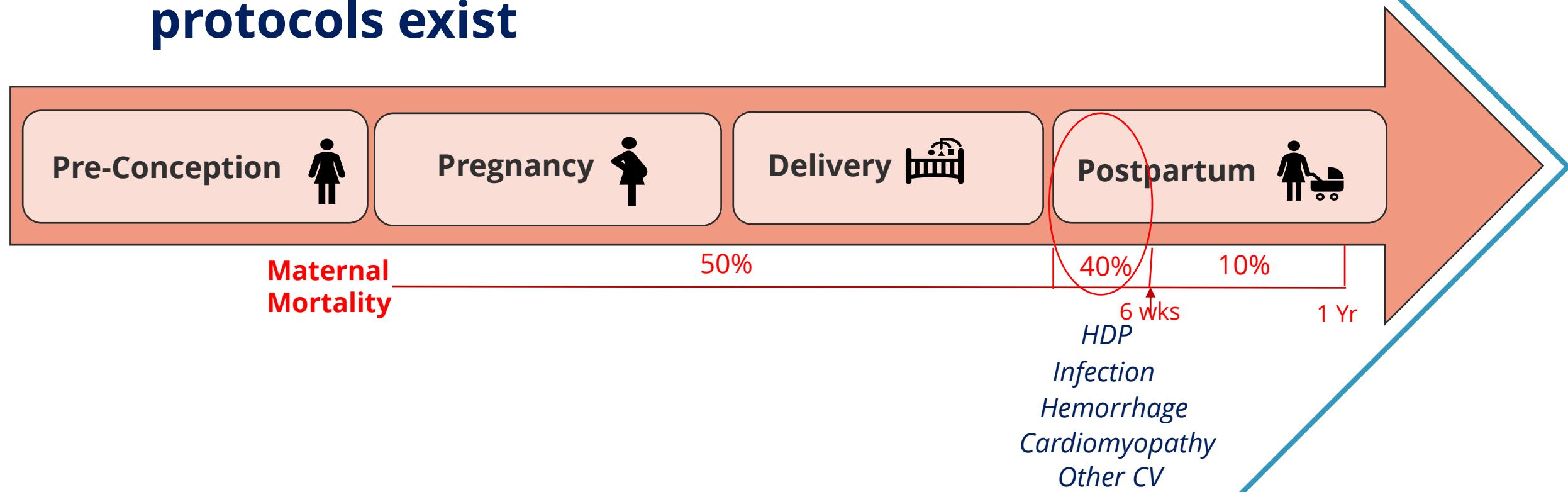
# Postpartum Care



# Postpartum Care



**Evidence-based treatments and care protocols exist**



# Postpartum Care



- **Postpartum care is limited, fragmented, and insufficient**
  - Transition from obstetric to primary care is critical
  - Many patients do not receive any postpartum care
  - Patients and providers may miss or dismiss warning signs
    - Signs and symptoms may overlap with other conditions
    - Patient may not mention and providers (OB, PC, ED) may not ask recent pregnancy status
- **Significant inequities persist in the receipt of timely and respectful postpartum care**
  - Provider bias
  - Language barrier
  - Cultural barriers
  - Social determinants of health

# Targeted PFA Cycle 2 2021



- **Research Question:** What is the comparative effectiveness of multicomponent strategies to improve early detection and timely care for complications up to six weeks postpartum for Black, AI/AN, Hispanic, rural, and low SES women?
  - Strategies may include increased patient/provider contact; standing orders and standardized protocols; patient education; provider education/training; reminder systems; home visits; telehealth; blood pressure monitoring models; care coordination
  - Particular interest in community-based and tailoring approaches
- Total commitment up to \$50M
  - Estimated number of studies: 4
  - Project duration: 5 years

## Q & A

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### Maternal Mortality



# **Closing Remarks/Adjourn**

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**Kari Gali**  
CEDS Chair

**Julie Eller**  
CEDS Co-Chair



# Thank you!

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