

# Clinical Effectiveness and Decision Science

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## PCORI Virtual Advisory Panel Meeting

November 10, 2020 | 1:50 pm–5 pm ET



# Conflict of Interest Statement



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

If this Committee 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.

# Welcome & Introductions

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**Bridget Gaglio, PhD, MPH**

Senior Program Officer, CEDS

**Cornell Wright, MPA**

CEDS Advisory Panel Chair

**Lawrence Goldberg, MD**

CEDS Advisory Panel Co-Chair



# CEDS Panel Chair and Co-Chair

## **Cornell Wright, MPA**

Executive Director, NC Office of Minority Health Disparities  
NC Department of Health and Human Services  
Representation: Policy Makers  
CEDS Advisory Panel Chair



## **Lawrence Goldberg, MD**

Psychiatrist Surveyor, The Joint Commission  
Representation: Clinicians  
CEDS Advisory Panel Co-Chair



# Agenda (1:50 pm-5 pm ET)

Time	Duration	Activity
1:50 pm ET	(20 min)	<b>Welcome and Introductions</b>
2:10 pm ET	(20 min)	<b>Acknowledgements for Panel Members Rolling Off</b>
2:30 pm ET	(15 min)	<b>CEDS Panel Overview</b>
2:45 pm ET	(10 min)	<b>BREAK</b>
2:55 pm ET	(90 min)	<b>CEDS Priority Areas Overview</b>
4:25 pm ET	(30 min)	<b>Update on New National Priority Areas and Cost Provision</b>
4:55 pm ET	(5 min)	<b>Closing Remarks/Adjourn</b>

# CEDS Panel Members Spring-Fall 2020

- Andrew Rosenberg, JD, MP\*
- Cornell Wright, MPA (CHAIR)\*
- Helen Osborne, M.Ed., OTR/L\*
- Lawrence Goldberg, MD (CO-CHAIR)\*
- Melissa Hicks\*
- Nancy Blake, PhD, RN, NEA-BC, CCRN\*
- Neela Goswami, MD, MPH\*
- Robin Karlin, MS\*
- Ruth M. Parker, MD, MACP\*
- Sandi Smith, PhD\*
- Maureen White, MD, MS, MBA
- David Webster, MD, MBA
- Eric Cannon, PharmD, FAMCP
- Danielle Bargo, MSc
- Samantha Harden, PhD, RYT® 500
- Karen Giuliano, PhD, MBA, RN
- Kari Gali, DNP, APRN, PNP-BC
- William Bennett, MD, MS
- Helen M. Beady, EdD, MEd
- Joey Mattingly, PharmD, PhD, MBA
- Rick Rader, MD
- Adjoa Adofo Kyerematen, MS
- Mychal Weinert, BS
- Lisa Goldman Rosas, PhD, MPH
- Susan Johnson, MBA, MS
- Julie Eller, BS
- Michael Philbin, PhD

\*Panel Members whose terms ended Aug 2020

# Acknowledgements

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**Bridget Gaglio, PhD, MPH**

Senior Program Officer, CEDS



# Farewells

- Andrew Rosenberg, JD, MP
- Cornell Wright, MPA (CHAIR)
- Helen Osborne, M.Ed., OTR/L
- Lawrence Goldberg, MD (Co-CHAIR)
- Melissa Hicks
- Nancy Blake, PhD, RN, NEA-BC, CCRN
- Neela Goswami, MD, MPH
- Robin Karlin, MS
- Ruth M. Parker, MD, MACP
- Sandi Smith, PhD

**Thank you for your contributions  
to PCORI and your service on the  
CEDS Advisory Panel!**

*Thank  
you*





# CEDS Panel Overview

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**Cornell Wright, MPA**

CEDS Advisory Panel Chair

**Lawrence Goldberg, MD**

CEDS Advisory Panel Co-Chair

**BREAK**

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2:45 pm–2:55 pm ET

# CEDS Priority Areas Overview

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**Bridget Gaglio, PhD, MPH**

Senior Program Officer, CEDS

**Holly Ramsawh, PhD**

Senior Program Officer, CEDS

**Jason Gerson, PhD**

Senior Program Officer, CEDS



# Strategic Planning

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Identifying National Priorities



# Existing National Priorities (Adopted in 2012)

## 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.

## 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.

## 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.

## 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.

## Accelerating PCOR 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.

# Strategic Planning: Encompassing Complex Components



# Clinical Effectiveness and Decision Science

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Assessment of Prevention, Diagnosis, and  
Treatment Options

Communication and Dissemination Research

Accelerating PCOR and Methodological Research



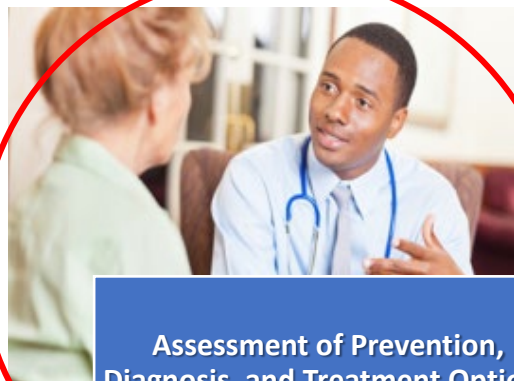
# Clinical Effectiveness and Decision Science



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.



# Overview of Clinical Effectiveness and Decision Science



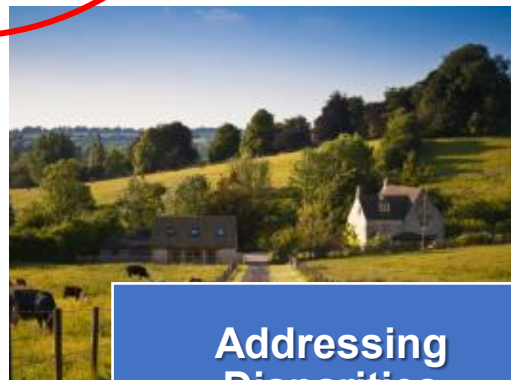
Assessment of Prevention,  
Diagnosis, and Treatment Options



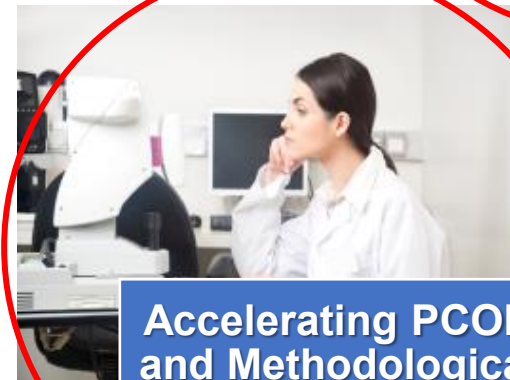
Improving  
Healthcare Systems



Communication &  
Dissemination  
Research



Addressing  
Disparities



Accelerating PCOR  
and Methodological  
Research

# Assessment of Prevention, Diagnosis, and Treatment Options (APDTO)

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Holly Ramsawh, CEDS



# Clinical Effectiveness and Decision Science



The **Clinical Effectiveness and Decision Science Program** is thus responsible for addressing three of PCORI's five National Priorities for Research by managing projects in the following areas:

- **Assessment of Prevention, Diagnosis, and Treatment Options:** Addresses gaps in the current evidence base across a broad range of clinical conditions and patient populations by comparing the outcomes of two or more healthcare interventions that are in widespread use or known to be effective.
- **Communication and Dissemination Research:** Addresses critical knowledge gaps in the communication and dissemination of research results to patients and caregivers by advancing the understanding of effective approaches to shared decision making between patients and their providers.
- **Accelerating Patient-Centered Outcomes Research and Methodological Research:** Focuses on improving methods for the design and conduct of clinical studies, thereby improving PCORI's and the nation's capacity to conduct high-quality, patient-centered CER and is complementary to the work of the PCORI Methodology Committee.

# Additional PCORI National Priorities



The remaining two National Priorities for Research are managed under the **Healthcare Delivery and Disparities Research (HDDR)** Program:

- **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.
- **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.

# APDTO Portfolio

## Program Overview



- Cycles: Cycle 1 2020 is the 20th release
- Funds Available: Historically, up to \$32M per cycle and up to \$2M in direct costs per project
- Duration: Typically 36 months
- Recent Addition: Small and Large study mechanisms (\$2M direct costs, 3-year max. duration; \$5M direct costs, 4-year max. duration)
- Projects Awarded: 203 through Cycle 2 2019
- Funds Awarded: Roughly \$743M through Cycle 2 2019
- Award amounts: ~\$438,751– \$20M in total costs
  - Median total costs of ~\$2,109,999.80
- DFRRs submitted: 96 as of 06/08/2020

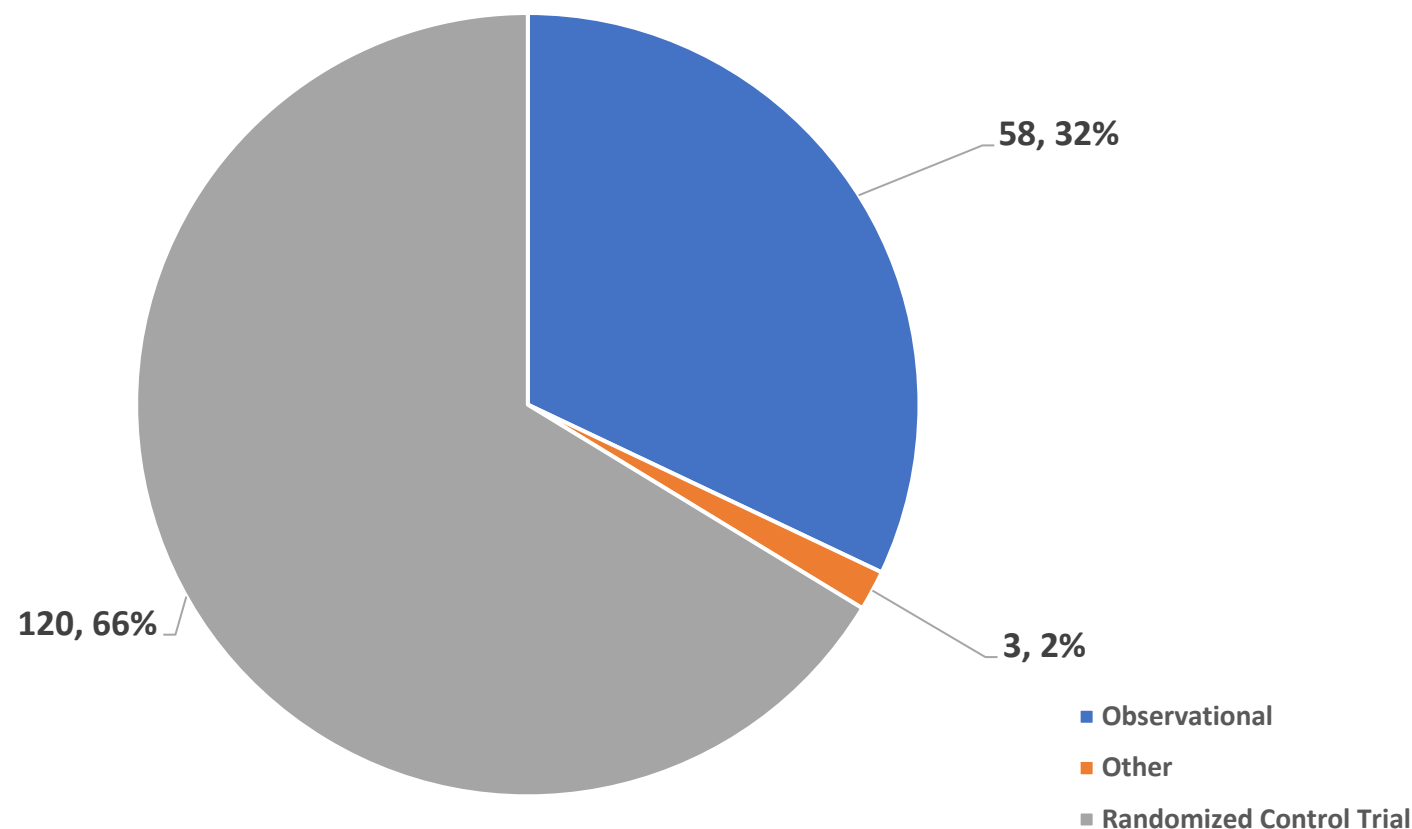
# APDTO Portfolio

## Primary Study Design

### For the RCTs

- Crossover designs: 1
- Cluster RCTs: 18
- Individual-level RCTs: 101
  - Individual RCT planned sample sizes range from 136 to 65,000

Study Designs of Award Projects



# Snapshot of CEDS Funded Projects

**Number of projects:**

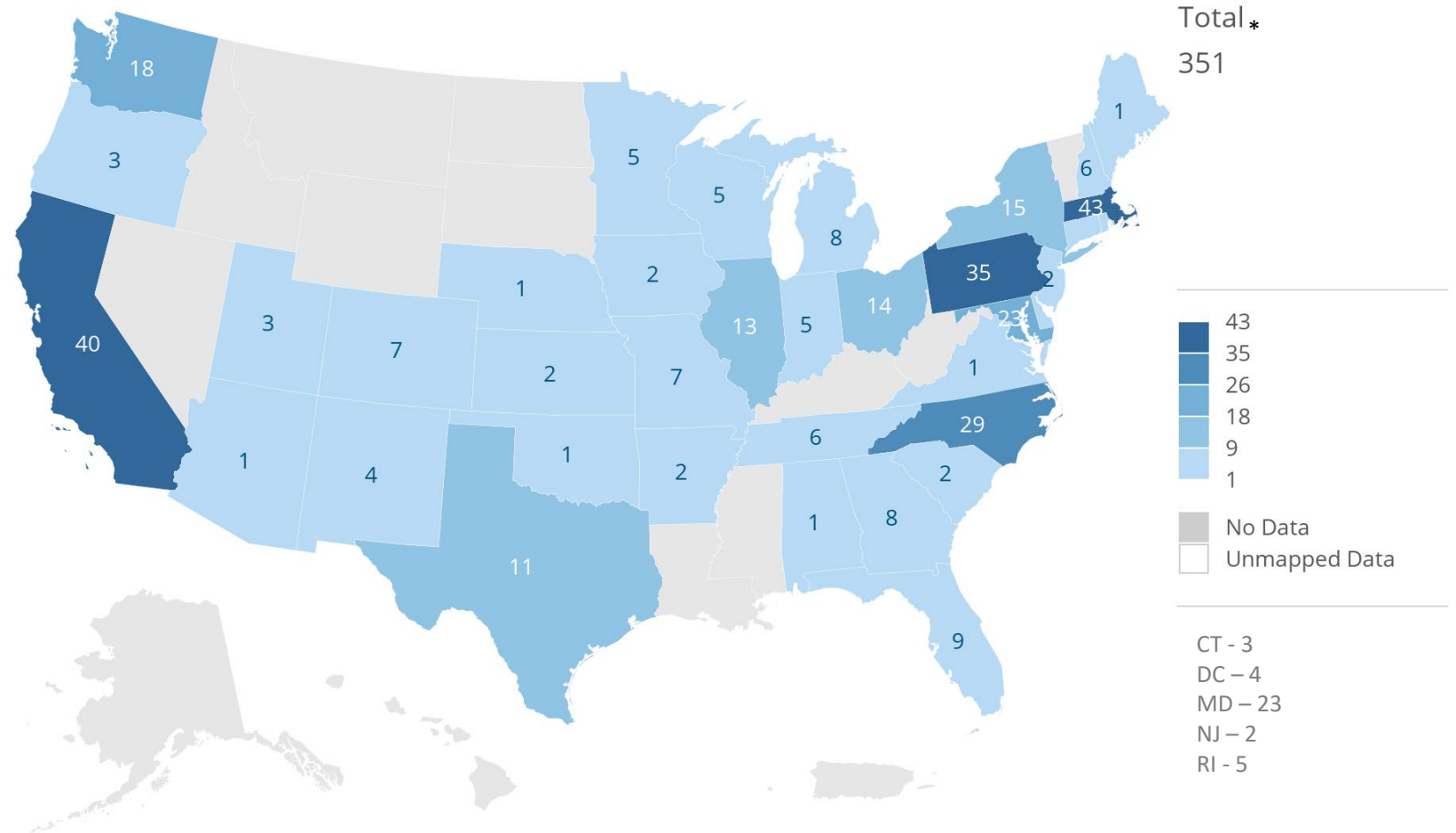
351

**Amount awarded:**

\$968.3 million

**Number of states where  
we are funding research:**

39 states and 4 countries  
(England, Sweden, Italy, &  
Canada)



+As of May 2020. Includes 4 studies funded England, Sweden, Italy, Canada

# National Priority: Assessment of Prevention, Diagnosis, and Treatment Options

## Portfolio Snapshot and Highlighted Results



# 203

Studies

### Spotlight on Research Topics

- Clinical Strategies for Managing and **Reducing Long-Term Opioid Use for Chronic Pain**
- New Oral Anticoagulants (NOACs) in the Extended Treatment of **Venous Thromboembolic Disease**
- Treatment of **Multiple Sclerosis**

Over

# 400

Results Publications

Including 120 CER Findings

### Highlighted Results



**Published in JAMA:** Association Between Radiation Therapy, Surgery, or Observation for **Localized Prostate Cancer** and Patient-Reported Outcomes After 3 Years



*Cited in UpToDate, Clinical Practice Guidelines, DynaMed Plus*



**Published in JAMA Internal:** Glucose Self-monitoring in **Non-Insulin-Treated Patients With Type 2 Diabetes** in Primary Care Settings: A Randomized Trial



*Cited in UpToDate & Clinical Practice Guidelines*



# National Priority: Assessment of Prevention, Diagnosis, and Treatment Options

## Highlighted Uptake

26

Citations in  
UpToDate

### Highlighted UpToDate Topics:

- Galactosemia: Management and complications
- Overview of approach to prostate cancer survivors
- Disease-modifying treatment of relapsing-remitting multiple sclerosis in adults
- Self-monitoring of blood glucose in management of adults with diabetes mellitus

7

Citations in Policy  
Documents

### Highlighted Policy Documents:

- WHO technical specifications for automated non-invasive blood pressure measuring devices with cuff
  - *World Health Organization*
- Harnessing Evidence and Experience to Change Culture: A Guiding Framework for Patient and Family Engaged Care
  - *National Academy of Medicine*

18

Citations in  
Clinical Practice  
Guidelines

### Highlighted Guidelines:



Diabetes Technology: Review of the 2019 American Diabetes Association Standards of Medical Care in Diabetes  
*American Diabetes Association*

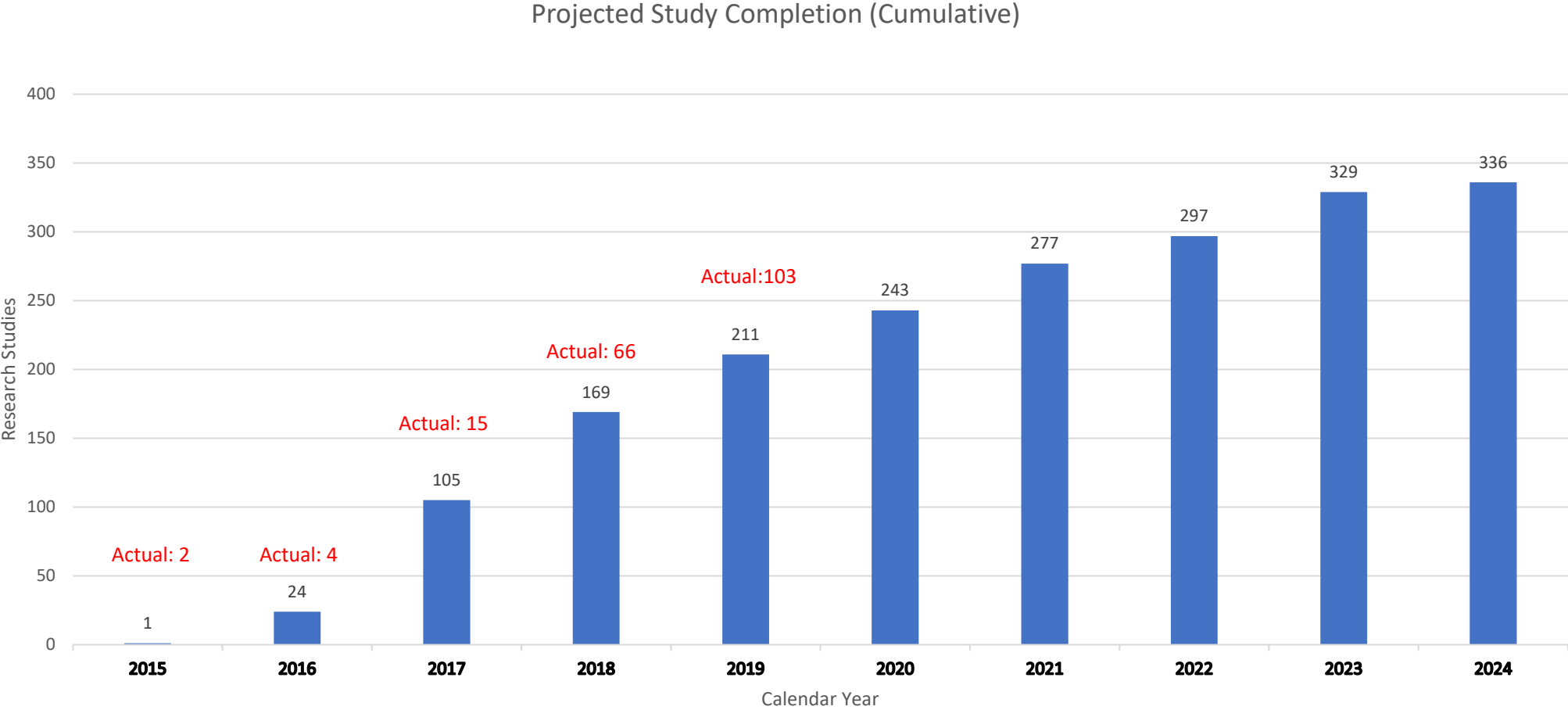


Guidelines for the care and treatment of chronic hepatitis C virus infection  
*World Health Organization*



Bone and Joint Infection Guidelines  
*The European Society for Paediatric Infectious Diseases*

# Projected CEDS Research Study Completion Through FY-2025



- Figure includes CEDS studies through Cycle 2 2019 for which there are DFRR due dates available (336 of the total 351 studies).
- Actual posting dates are based on publication of primary results and/or abstracts posted to PCORI.org.
- This figure does not include estimates for future extensions, only those already approved.

# Examples of Completed Projects in APDTO

## Drug vs. Drug Comparisons

- Comparative Effectiveness of Broad- versus Narrow-Spectrum Antibiotics for Acute Respiratory Tract Infections in Children
- CE-1304-7279

## Mental Health

- Choosing Options for Insomnia in Cancer Effectively (CHOICE): A Comparative Effectiveness Trial of Acupuncture and Cognitive Behavior Therapy
- CER-1403-14292-IC

## Cancer

- Comparing Surgeries for Women Who Have Both Cancer of the Uterus and Bladder Problems
- CER-1409-22034

## Diabetes

- Does Daily Self-Monitoring of Blood Sugar Levels Improve Blood Sugar Control and Quality of Life for Patients with Type 2 Diabetes Who Do Not Use Insulin? -- The Monitor Trial
- CE-12-11-4980

## Rare Disease

- Developmental Outcomes in Children with Duarte Galactosemia
- CER-1408-19941

# Questions/Discussion

- Thank you!

# Communication and Dissemination Research Portfolio Overview

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Bridget Gaglio, CEDS



# CDR Portfolio

## Program Overview



- Cycles: Cycle 1 2020 is the 20th release
- Funds Available: Historically, up to \$8M per cycle and up to \$2M in direct costs per project
- Duration: Typically 36 months
- Projects Awarded: 54 through Cycle 2 2019
- Funds Awarded: Roughly \$117M through Cycle 2 2019
- Award amounts: ~\$697,104–\$8,009,505 in total costs
  - Median total costs of ~\$2,052,893
- Draft Final Research Reports submitted: 36 as of 06/08/2020

# Examples of Completed Projects in CDR

## Communication Strategies

- Comparative effectiveness of encounter decision aids for early-stage breast cancer across socioeconomic strata

## Dissemination Strategies

- CPR education for families of cardiac patients before hospital discharge: comparing methods for real-world dissemination

## Explaining Uncertainty

- Measuring the impact of providing personalized risk information to patients and their providers

- Communication focused proposals tend to focus on decision aids.
- Dissemination–inclusion of effectiveness-implementation hybrid study designs.
- Dissemination and implementation science is advancing but is still a fairly new field.
- De-implementation of interventions that are ineffective, unproven, low-value, or harmful is another area for potential future focus.
- CDR has always been disease/condition agnostic. This priority area is cross-cutting of the other priority areas.



# Questions/Discussion

- Thank you!

# Methods Awards Portfolio Overview

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Jason Gerson, CEDS



# Methods Portfolio

## Program Overview



- Cycles: Cycle 3 2020 is the 20th release
- Funds Available: Historically, up to \$12M per cycle and up to \$750K in direct costs per project
- Duration: Typically 36 months
- Projects Awarded: 117 through Cycle 2 2019
- Funds Awarded: Roughly \$108M through Cycle 2 2019
- Award amounts: ~\$123,972 – \$1,761,691 in total costs
  - Median total costs of ~\$1,007,806
- DFRRs submitted: 75 as of 06/08/2020
- 64 LOIs received for Cycle 3 2020
- 8 Methods COVID Enhancement Awards

# Methods Portfolio

## *Research Areas of Interest (RAIs)*



**Current RAIs:** (see pp. 2-3 of Methods PFA: <https://www.pcori.org/sites/default/files/PCORI-2020-Cycle-3-Methods-PFA.pdf>)

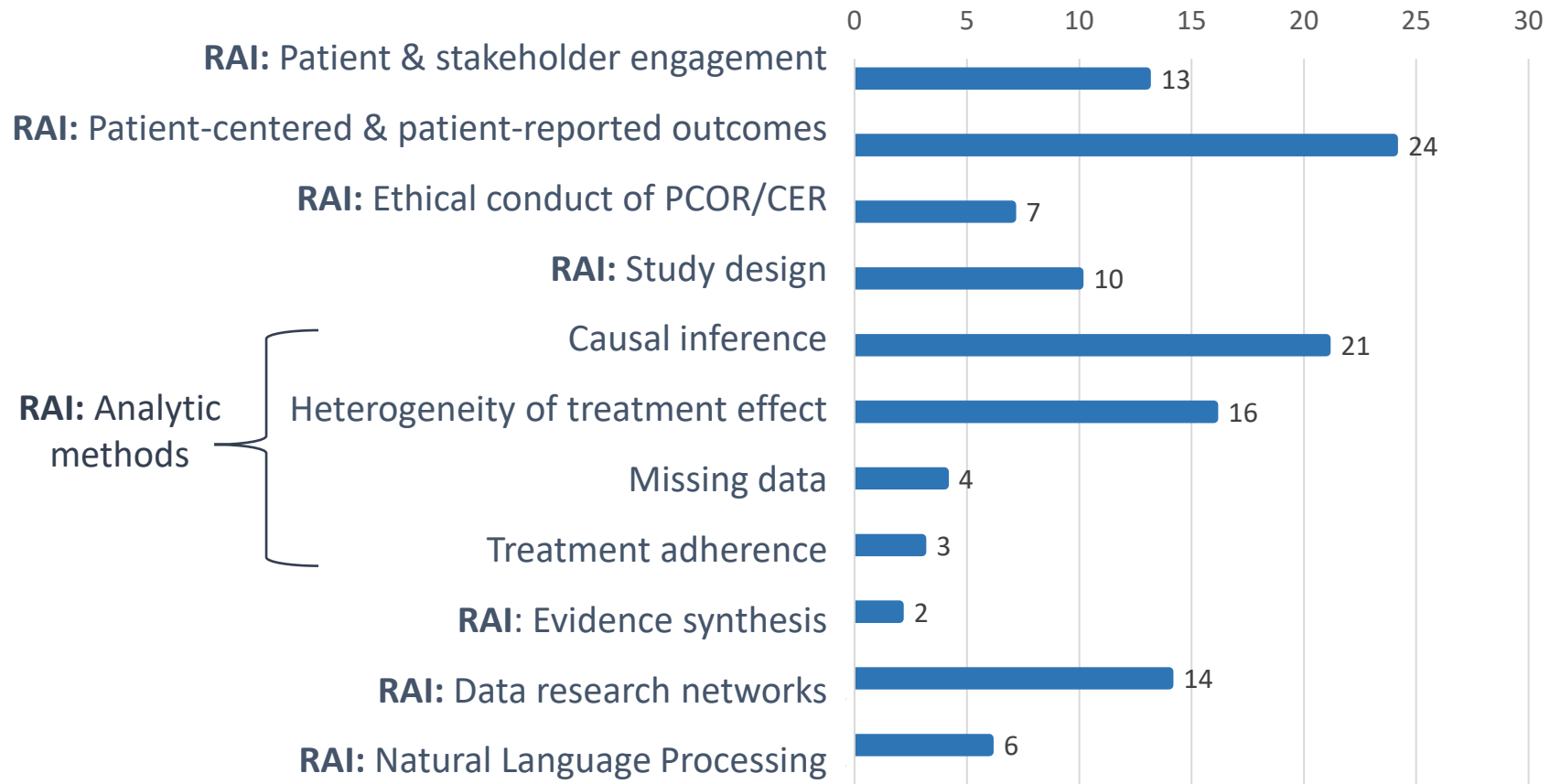
1. Methods to Improve Study Design
2. Methods to Support Data Research Networks
3. Methods to Improve Use of Artificial Intelligence and Machine Learning in Clinical Research (new as of 20C3)
4. Methods Related to Ethical and Human Subjects Protection

### **Legacy RAIs:**

- Methods for Patient and Stakeholder Engagement
- Methods for Evidence Synthesis
- Methods for Patient-Centered Outcomes (PCOs) and Patient-Reported Outcomes (PROs)
- Methods to Improve Validity and Efficiency of Analyses (Analytic Methods)
- Methods to Improve the Use of Natural Language Processing (NLP)

# Methods Program Portfolio\*

117 projects funded through Cycle 2 2019<sup>^</sup>



\*Some projects are classified into >1 RAI in this graph

<sup>^</sup>1 additional project funded under the Communication & Dissemination Research (CDR) PFA

# Examples of Ongoing and Completed Projects in Methods

## Methods Related to Ethical and Human Subjects Protection

- Demonstrating Respect and Acceptable Consent Strategies: What Matters to Patients in PCOR?
- ME-1310-07763

## Methods to Improve Study Design & Analytic Methods

- Methods to Assess the Effect of Dynamic Treatment Regimens Using Electronic Health Records
- ME-1403-12506

## Methods to Support Data Research Networks

- Efficient Distributed Learning Framework for Integrating Evidence in Clinical Research Networks
- ME-2019C3-18315

## Methods to Improve the Use of Natural Language Processing

- Natural Language Processing to Connect Social Determinants and Clinical Factors for Outcomes Research
- ME-2018C3-14754

# Study Design and Analytic Methods: Publications

- Looking at the publications from the ~50 studies in this part of the Methods portfolio, a few observations:
- All are publishing multiple manuscripts in statistics/methods journals.
  - *Biostatistics, Biometrics, Statistics in Medicine, Journal of the Royal Statistical Society, American Journal of Epidemiology, Contemporary Clinical Trials*
- Some are publishing single manuscripts in clinical research journals relevant to project aims – less technical version of methods manuscripts.
  - *BMJ, Stroke, AIDS and Behavior, Clinical Kidney Journal, Clinical Infectious Diseases, Journal of the American Heart Association*
- A few report publications in clinical research journals where the PCORI-funded methods have been used in the design/conduct of trials/obs studies.

# COVID-19 Enhancements: Methods Awards



- Additions to existing aims, or an adjunct project that has some relationship to the original award, including a new aim (or aims) designed to produce useful knowledge related to COVID-19
  - Methods awardees were encouraged to apply if enhancements were COVID related and would improve the rigor of collecting data, analyzing data, or produce immediately applicable tools.
- 12 months/\$500K
- 134 research applications → 20 Methods applications → 8 Methods awards
  - 2 funded proposals included clinical questions, using methods developed with PCORI funding to analyze effectiveness of drugs on the prevention or treatment of COVID-19. Proposals include clinical outcomes (e.g., hospitalizations, length of stay, mortality).
  - Clinical predictive models
    - Hospitalized patients with COVID-19 -whether they should be “full treat” versus “do not intubate.”
    - Nursing homes that are likely to experience a COVID-19 outbreak, nursing home residents who are likely to develop COVID-19, and nursing home residents who are likely to experience unfavorable outcomes after the diagnosis of COVID-19.



# Questions/Discussion

- Thank you!

# Update on New National Priority Areas and Cost Provision

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**Els Houtsmuller, PhD**

Associate Director, HDDR

**Amanda Barbeau, MPH**

Program Associate, CEDS

**Andrew Hu, MPP**

Director, Engagement

# Maternal Mortality and Morbidity (MMM)

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Els Houtsmuller, HDDR



# Putting our Mandate into Action

- Reauthorization language included two research priorities:
  - Maternal morbidity and mortality (MMM)
  - Intellectual and/or developmental disabilities (IDD)

## Hear from Dr. Nakela Cook in her recent blog

### Formulating Our Approach to New Priority Research Areas

**Date:** September 8, 2020

**Blog Topics:** [Executive Director's Blogs](#),  
[Funding Awards](#),  
[Research](#)

As part of last winter's legislation that reauthorized PCORI's funding for 10 years, Congress included two new research priority areas for PCORI to address: strategies for improving maternal mortality, and improving health for individuals with intellectual and/or developmental disabilities (IDD).

These areas are of critical importance for PCORI given the long-standing health challenges faced by those affected and the opportunities that PCORI's approach to *research done differently* can contribute to meaningful health improvements. We are committed to a multipronged approach to funding research to address a variety of issues related to these two topics over the next decade.

#### Addressing Maternal Morbidity and Mortality

The United States consistently ranks near the bottom among high-income



# Addressing PCORI's New Research Priorities



## Stakeholder Engagement Literature Reviews

### CER AWARDS

- *Broads ✓*
- *Phased large awards ✓*
- *Pragmatic clinical studies*
- *Targeted funding announcements*

### EVIDENCE SYNTHESIS

- *Systematic reviews*
- *Rapid reviews*
- *Evidence maps and/or visualizations*

### ENGAGEMENT AWARDS

- *Capacity building ✓*
- *Stakeholder convening support ✓*
- *Dissemination*

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# Maternal Mortality and Morbidity

# PCORI's Current Special Areas of Emphasis



Up to \$30 million set aside for **each** of these topics, with available funding emphasizing:

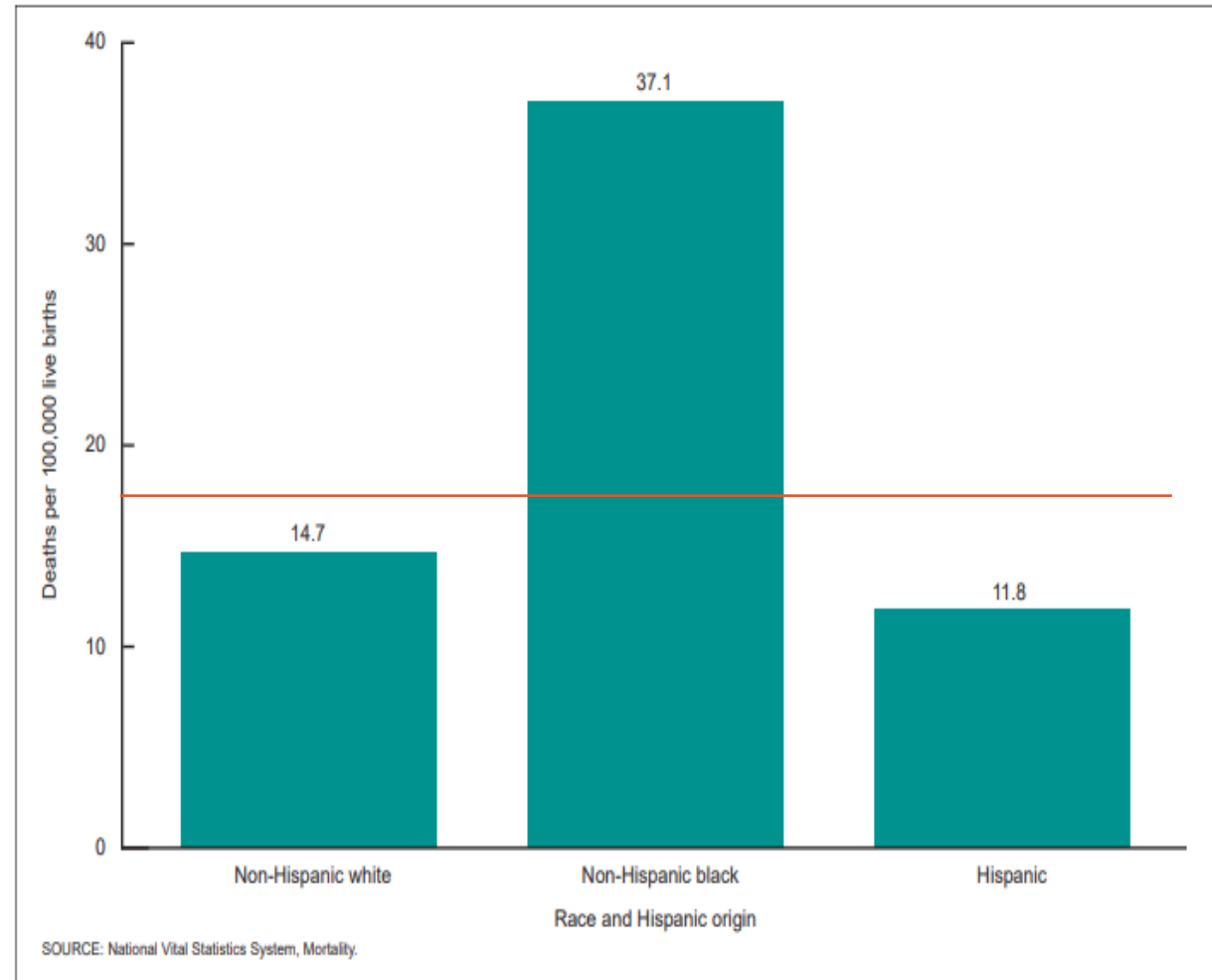
- Care and care transitions for individuals with intellectual and/or developmental disabilities growing into adulthood
- Person-centered maternal care for populations likely to experience the most significant disparities in care and/or outcomes

Please find additional details on the [PCORI website](#) and within the handout provided.

# Maternal Mortality: US Rates and Disparities

## 2017 Maternal Mortality Rates per 100,000 Live Births by Country

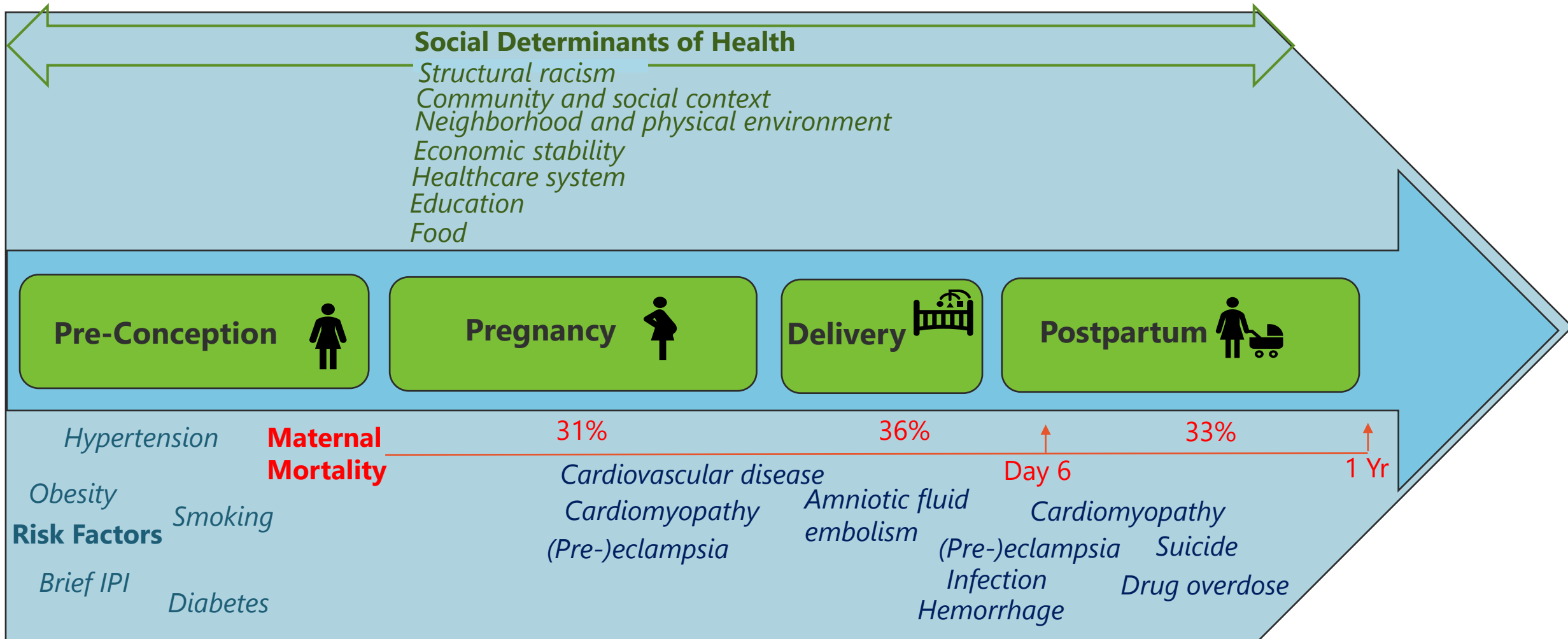
Norway	2
Italy	2
Finland	3
Greece	3
Denmark	4
Spain	4
Sweden	4
Iceland	4
Austria	5
Netherlands	5
Japan	5
Switzerland	5
Germany	7
US	19



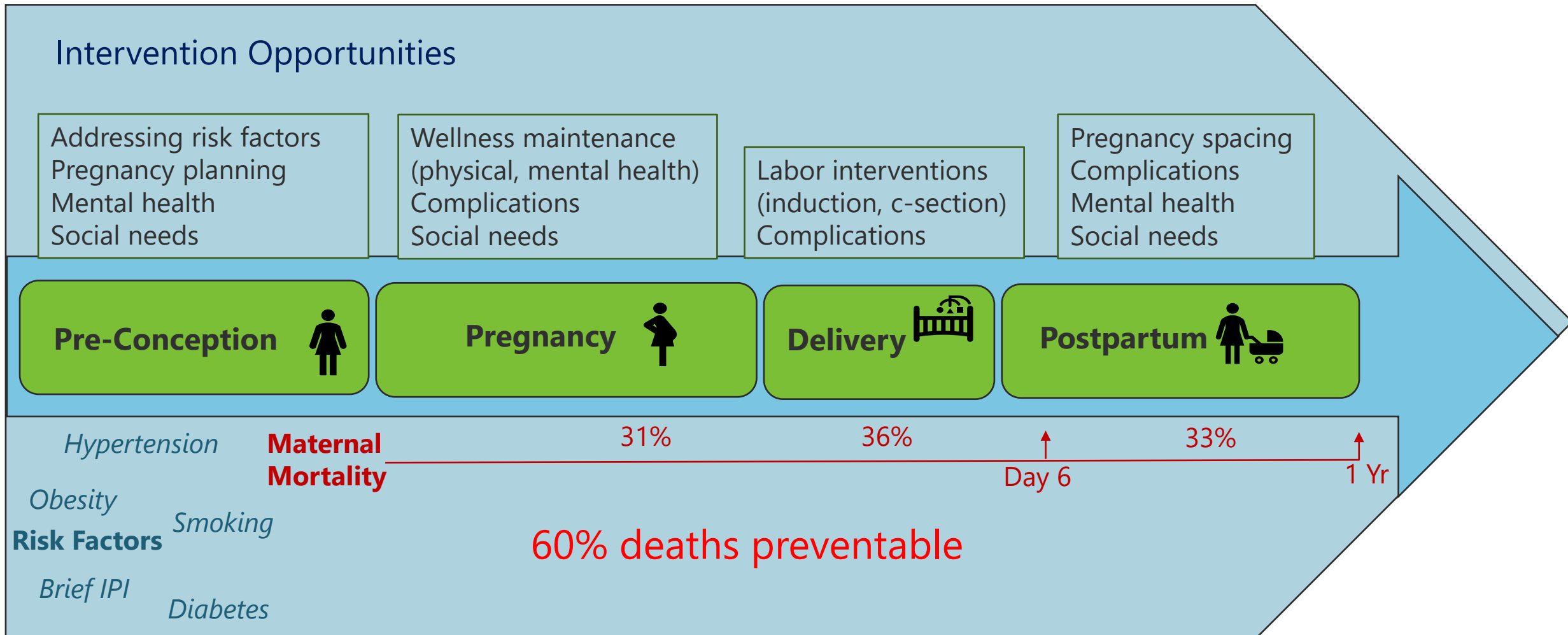
Maternal mortality rates, by single race and Hispanic origin: United States, 2018



# Maternal Mortality Framework: More than Just Pregnancy and Delivery



# Maternal Mortality Framework: Opportunities for Healthcare Intervention



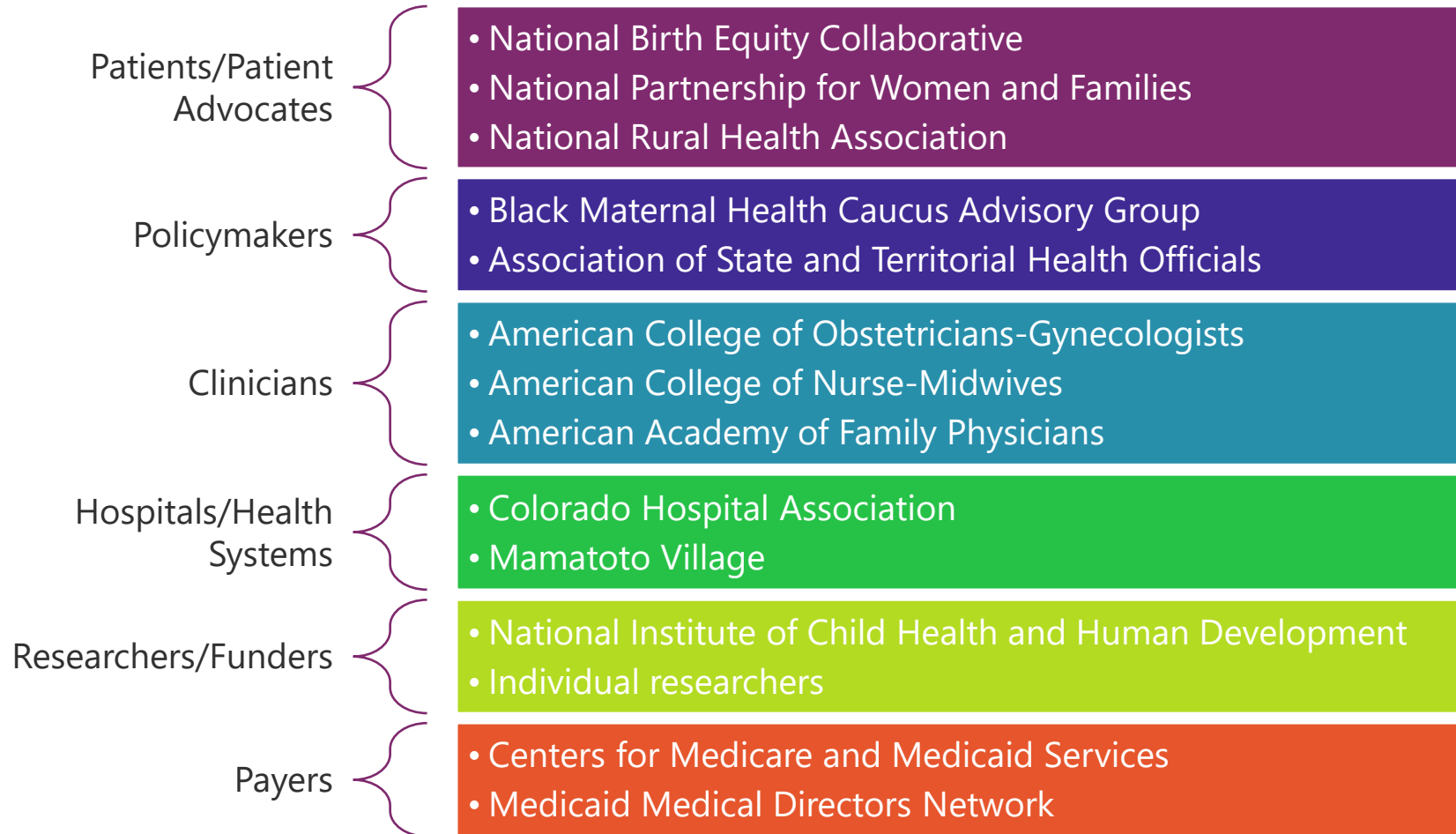
# Maternal Mortality

## Stakeholder Engagement to Date: A Sample

- A few organizations that we've engaged with
- Not an exhaustive list

### Key Question for Panel

- Are there organizations we should consider?



# Intellectual and Developmental Disabilities (IDD)

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Amanda Barbeau, CEDS



# Intellectual and Developmental Disabilities



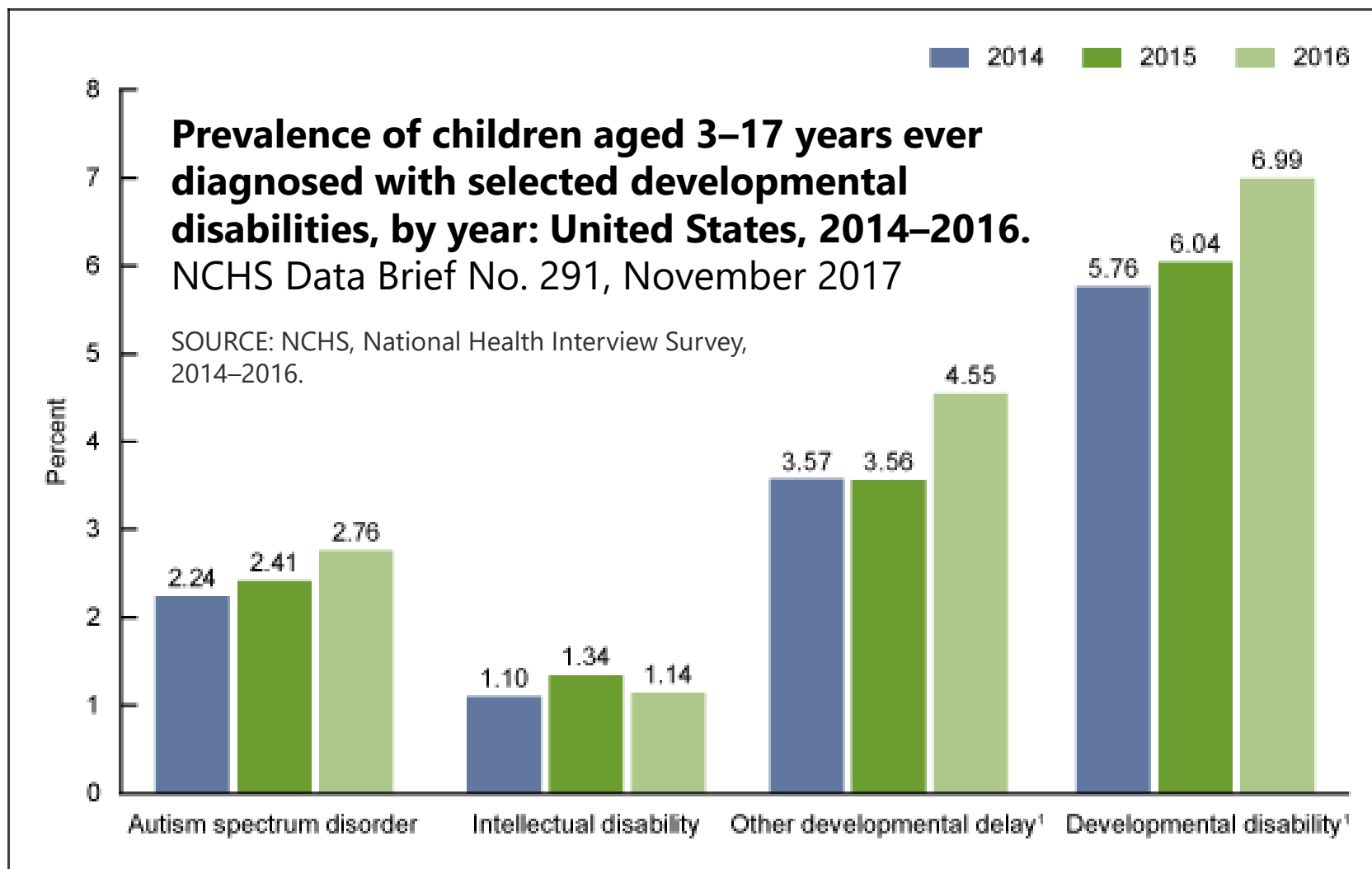
**Intellectual and developmental disabilities (IDD)** are disorders that are usually present at birth and that negatively affect the trajectory of the individual's physical, intellectual, and/or emotional development. [NICHD]

- **Intellectual disabilities** are characterized by significant limitations in both intellectual functioning and adaptive behavior. [AAIDD]
- **Developmental disabilities** are chronic and can be cognitive, physical or both. [AAIDD]

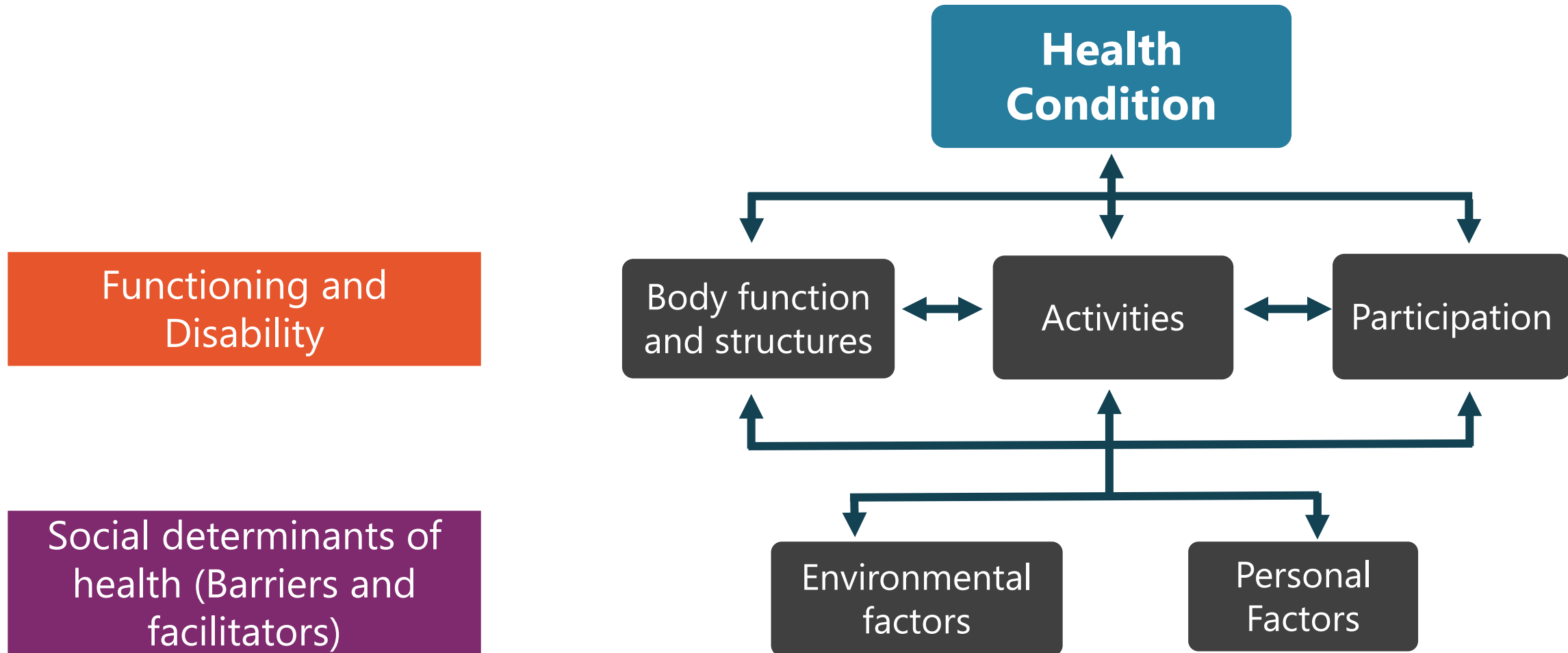
**Not all developmental disabilities include limitations in cognitive ability.**

People with IDD comprise a vulnerable population with poorer health status, shorter lifespan, and worse health care outcomes than the general population

- Additional challenges for populations already at risk for disparities



# Levers for Improving Health Outcomes for Individuals with IDD: ICF Model



# Intellectual and/or Developmental Disabilities

## Stakeholder Engagement to Date: A Sample

- A few organizations with whom we've engaged
- Not an exhaustive list

### Future question for Panel to consider:

- Are there organizations we should consider?



*PCORI hosted a multi-stakeholder town hall at the 2020 PCORI Annual meeting.*



# PCORI's Current Focus on IDD: Improving Care While Growing into Adulthood



Through the SAE funding mechanism, PCORI is homing in on a critical gap for individuals with IDD—the **transition from pediatric/adolescent care to adult care**.

- Adolescents with IDD often have **special healthcare needs** (SHCN).
- This healthcare transition is critical for adolescents with SHCN as they move from a child-oriented to an adult-oriented healthcare setting. **This adult setting has fewer systems supports, such as care planning and care coordination.**
- Compared to other patients with SHCN, individuals with IDD are less likely to report adequate support in their transition and to receive supports needed to direct their own care, and **more likely to incompletely transition** to adult care.
- **Incomplete transitions are associated with decreased receipt of routine care**, tests, and vaccinations and increased unmet physical/mental health and prescription needs.



# Defining Barriers, Seeking Solutions

- Inadequate transition planning
- Drop-off in services once people turn 21
- Insufficient adult providers who are willing and/or able to treat individuals with IDD

**Furthermore**, research has shown that racial and ethnic minority individuals with IDD are subject to even greater health disparities compared to their nondisabled peers.

# Defining Barriers, Seeking Solutions (cont.)

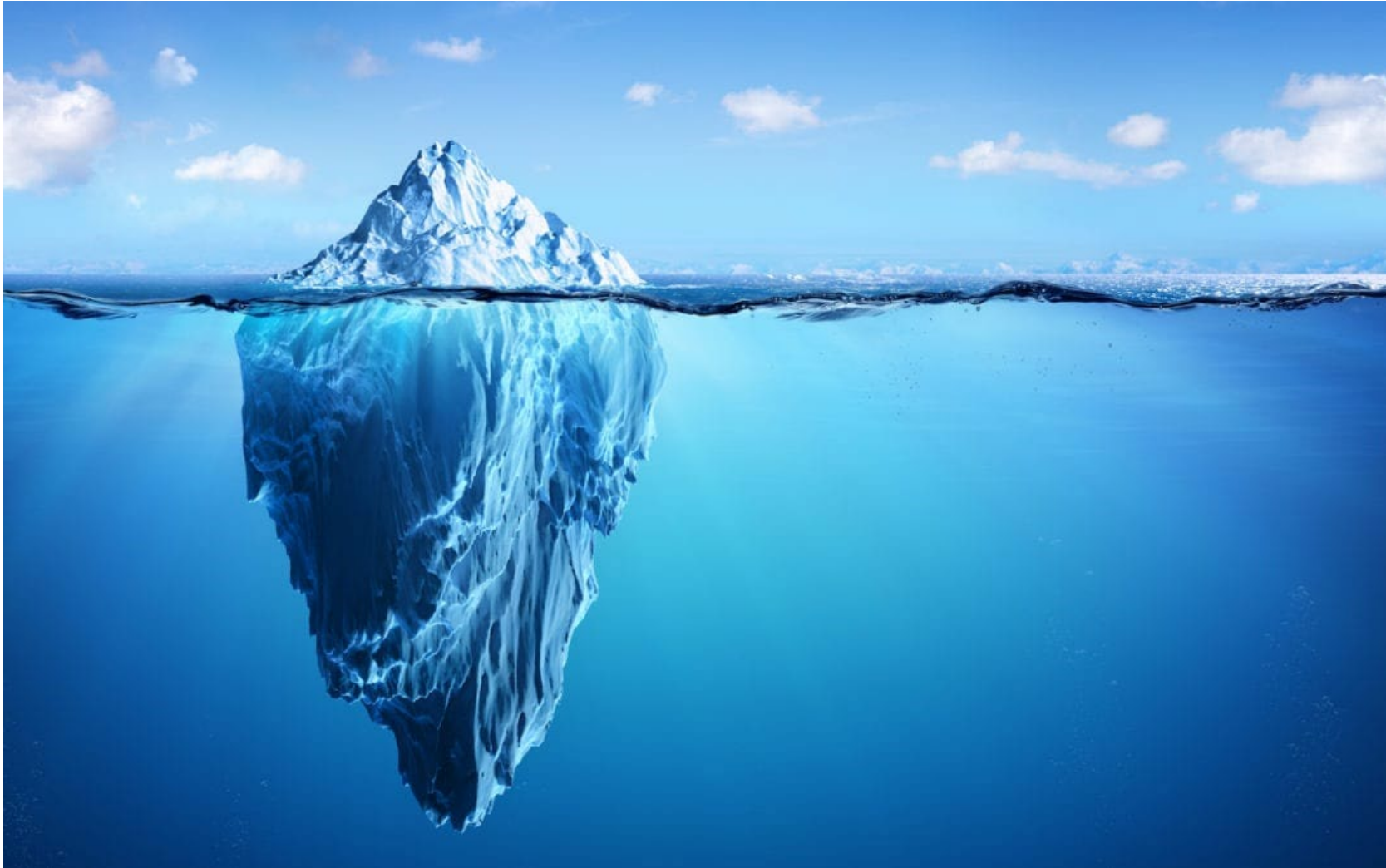
To address these barriers, PCORI initiated the SAE to solicit applications for **comparative effectiveness research** of interventions to optimize healthcare transitions from childhood to adulthood and the continuation of patient-centered physical and mental health care for individuals with IDD.

## Interventions may include:

- Care delivery models
- Person-centered transition planning
- Patient, family, caregiver, and provider support during the transfer of care
- Support for the continuation of general and specialty adult care
- Care coordination



# The Next 10 years



For questions, suggestions and thoughts related to how PCORI can focus on IDD in the future, please email Amanda Barbeau at [abarbeau@pcori.org](mailto:abarbeau@pcori.org)

**Thank you!**

# Cost Provision

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Andrew Hu, Engagement



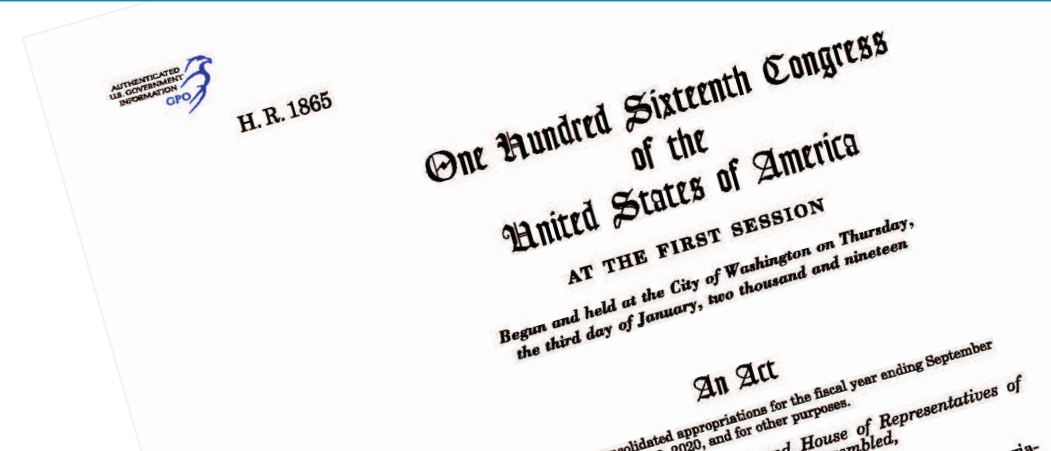
# Overview of New Statutory Authority

PCORI's reauthorizing legislation directs PCORI to capture, as appropriate, the full range of outcomes data in the course of our research studies.

This includes economic and cost data related to the utilization of healthcare services, but also outcomes and measures of cost and burden important to patients.

## Potential Burdens and Economic Impacts Include:

- Medical out-of-pocket costs, including health plan benefit and formulary design
- Nonmedical costs to the patient and family, including caregiving
- Effects on future costs of care
- Workplace productivity and absenteeism
- Healthcare utilization



# Overview of PCORI's Cost Data Implementation Proposal

## Pillar 1

- Providing guidance to principal investigators in future PFAs on how they should interpret this policy and incorporate it into their research proposals.
- **Timeline:** Final Principles and Guidance for Applicants by **February or March 2021**

## Pillar 2

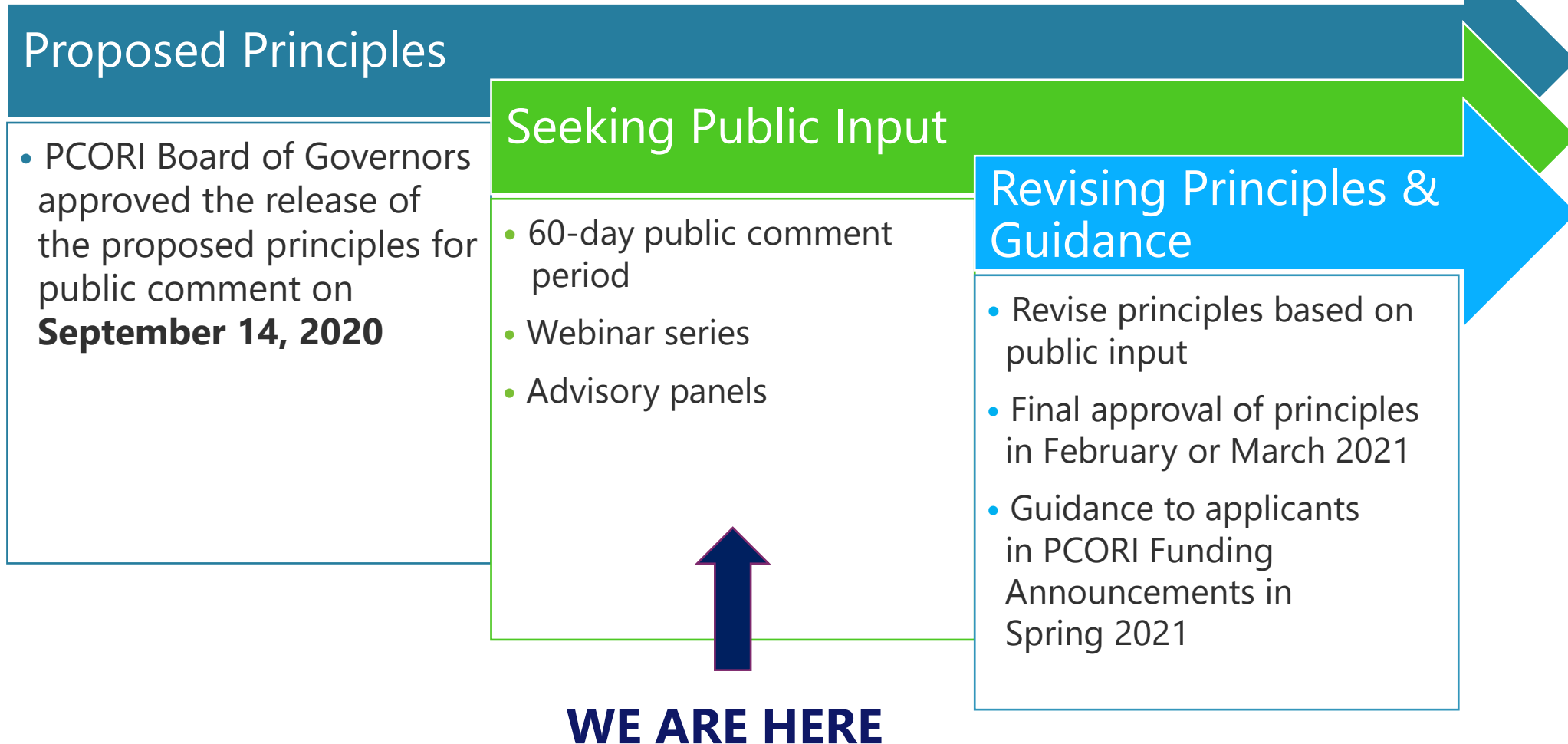
- Establishing methodology standards to further inform how PCORI-funded studies should capture relevant data.
- **Timeline:** Approximately **12 months** from the initiation of this process

## Pillar 3

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



# Progress Report – Pillar 1



# Proposed Principles for the Consideration of the Full Range of Outcomes Data

## What are the principles?

- These principles are a **high-level framework** to describe PCORI's interpretation of the new mandate to collect cost burden and economic impact data.

## Why do we need them?

- To provide the public and potential applicants with an understanding of how PCORI interprets the mandate.

## How will they be used?

- These principles will serve as a point of reference for PCORI as a basis for developing guidance to potential applicants and updating PCORI's Methodology Standards.
- These principles should not be viewed as standards and methods.

# Proposed Principles

## ***Identifying Outcomes Important to Patients***

- **Principle #1:** PCORI-funded research may consider the full range of outcomes *important to patients and caregivers*, including burdens and economic impacts.

## ***Identifying Outcomes Important to Stakeholders***

- **Principle #2:** PCORI-funded research may consider the full range of outcomes *relevant to other stakeholders*, when these outcomes have a near-term or longer-term impact on patients.

## ***Criteria Regarding the Collection of Data***

- **Principle #3:** The collection of data on burdens and economic impacts of treatment options must be appropriate and relevant to the clinical aims of the study.

## ***Consideration of Economic Analysis***

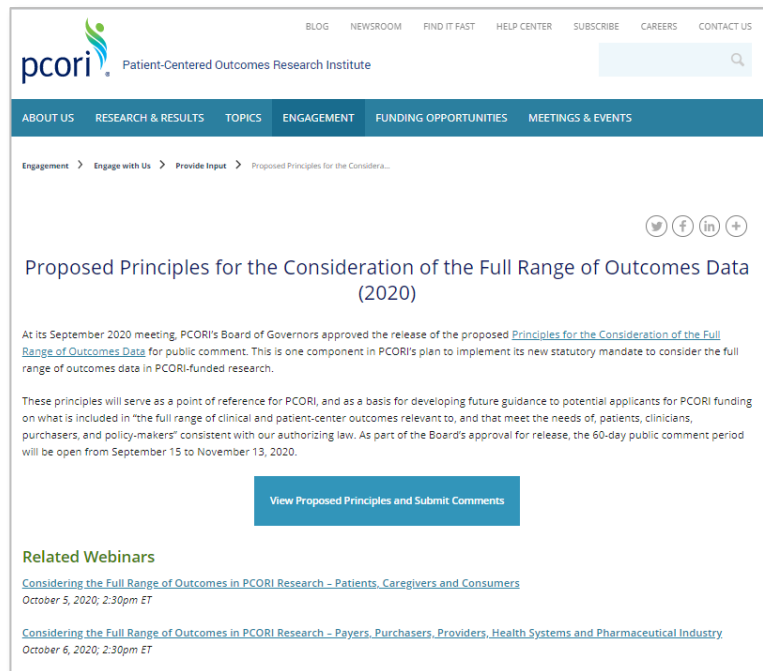
- **Principle #4:** Beyond the collection of burden and economic impact data, PCORI may support the conduct of certain types of economic analyses as part of a funded research study, to enhance the relevance and value of this information to health care decision-makers.

# Themes of Input Received

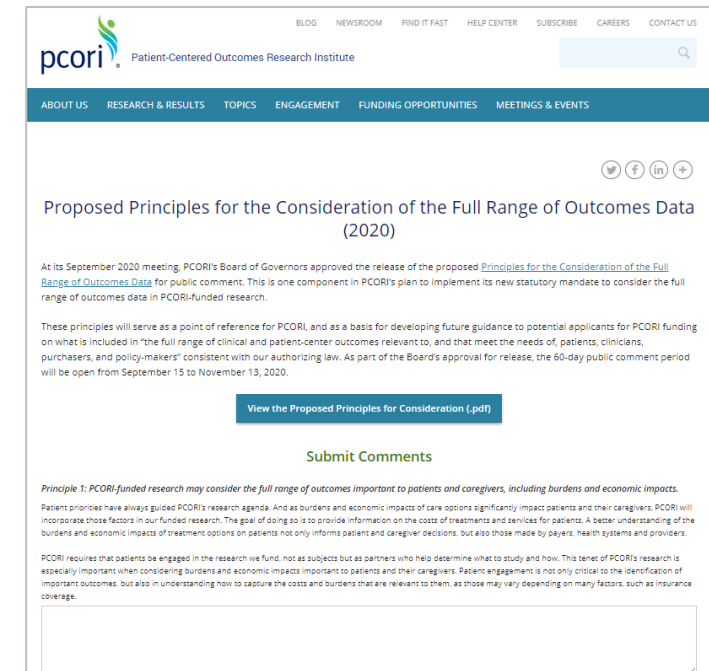
- Broad **support** for the consideration of costs and economic impact data in PCORI research
- Ensure a **patient-centered and holistic** approach to the consideration of costs
- Consider the cost burdens and impacts from a **societal and community** level
- Helpful to capture **implementation or program costs**
- Having patient-centered cost/impact data can help in **value-based payment** models

# References & Resources

- [Proposed Principles for the Consideration of the Full Range of Outcomes Data \(\*Landing Page\*\)](#)



- [Proposed Principles for the Consideration of the Full Range of Outcomes Data \(\*Public Comment Webform\*\)](#)



# Closing Remarks/Adjourn

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**Cornell Wright, MPP**

CEDS Chair

**Lawrence Goldberg, MD**

CEDS Co-Chair



**Thank you!**

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