

Clinical Effectiveness and Decision Science

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

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

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

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

Cornell Wright, MPA

CEDS Advisory Panel Chair

Lawrence Goldberg, MD

CEDS Advisory Panel Co-Chair

BREAK

2:45 pm–2:55 pm ET



CEDS Priority Areas Overview

Bridget Gaglio, PhD, MPH

Senior Program Officer, CEDS

Holly Ramsawh, PhD

Senior Program Officer, CEDS

Jason Gerson, PhD

Senior Program Officer, CEDS



Strategic Planning

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

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



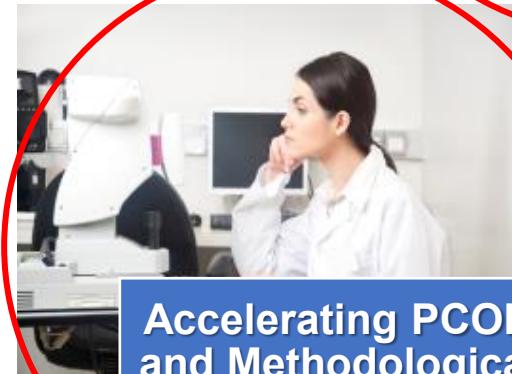
Improving
Healthcare Systems



Communication &
Dissemination
Research



Addressing
Disparities



Accelerating PCOR
and Methodological
Research

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

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

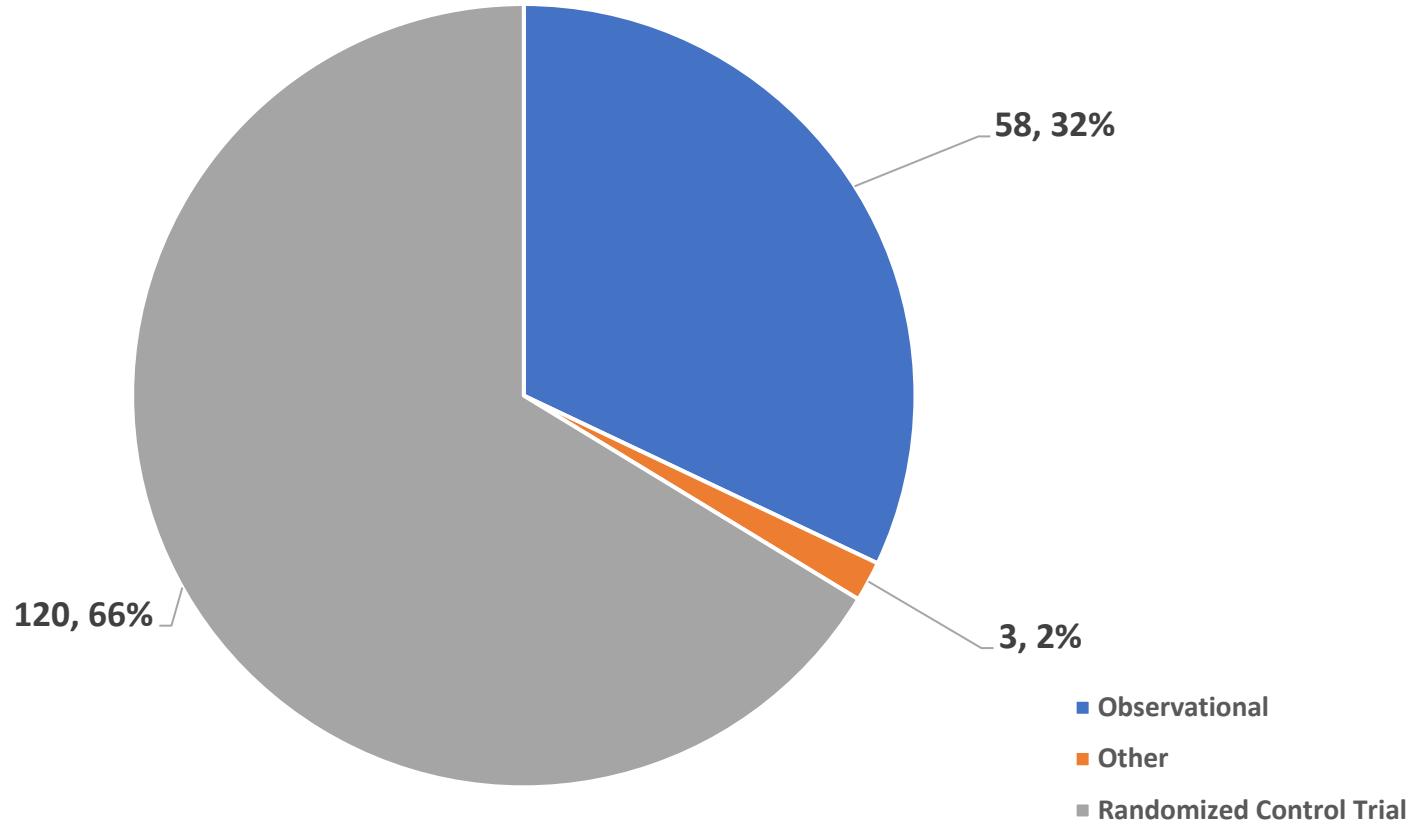
Primary Study Design



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:

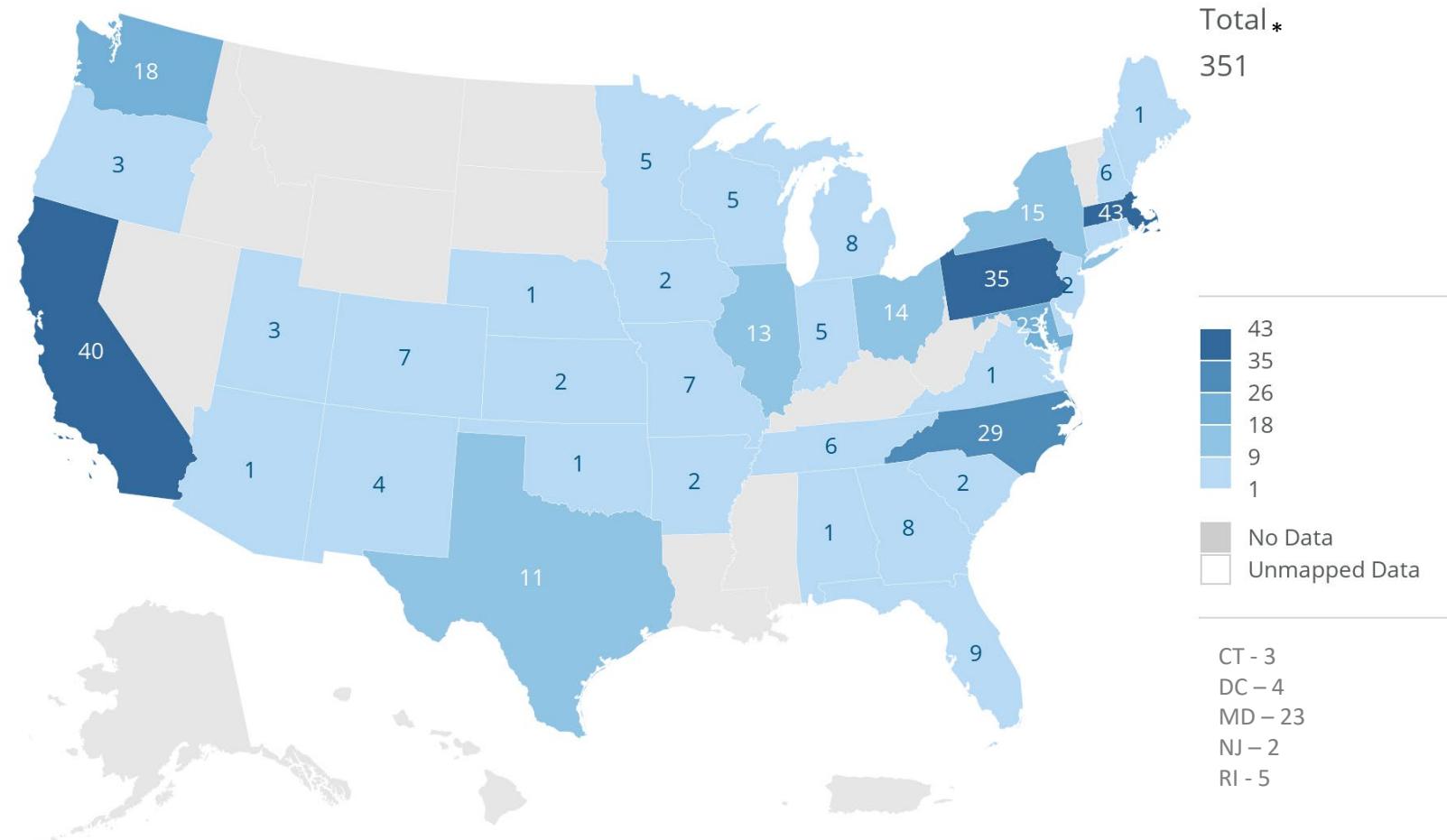
351

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

Over

400

Results Publications

Including 120 CER Findings

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

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

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*

7

Citations in Policy
Documents

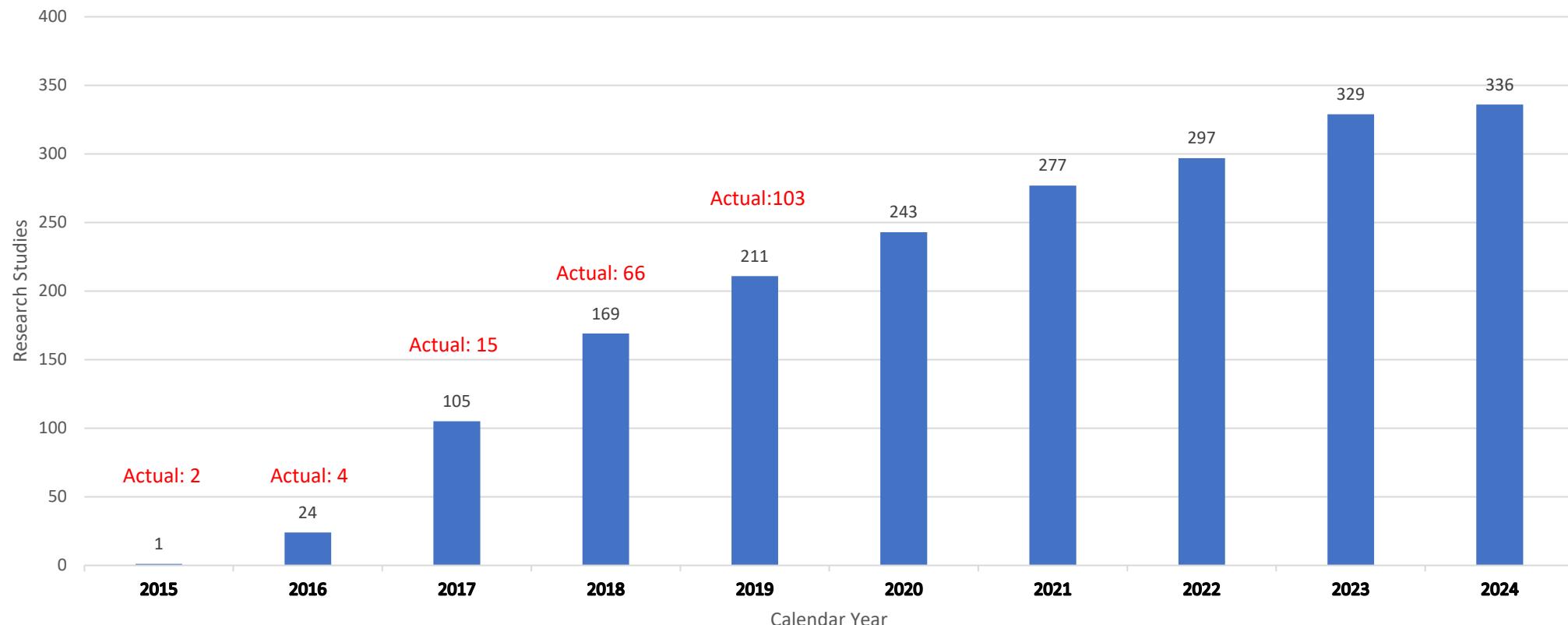
Highlighted Policy Documents:

- 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*

Projected CEDS Research Study Completion Through FY-2025



Projected Study Completion (Cumulative)



- 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

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

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>)

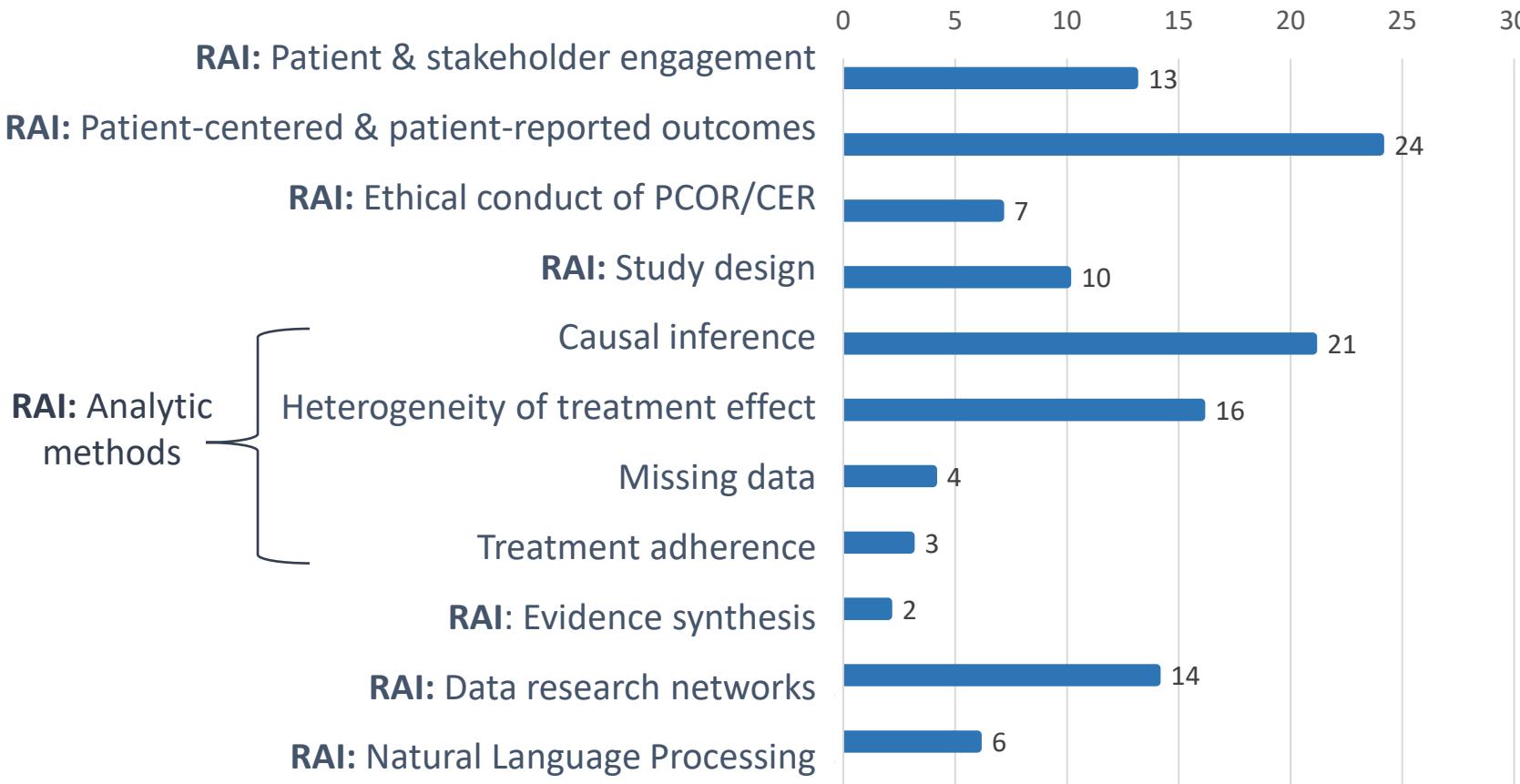
1. Methods to Improve Study Design
2. Methods to Support Data Research Networks
3. Methods to Improve Use of Artificial Intelligence and Machine Learning in Clinical Research (new as of 20C3)
4. Methods Related to Ethical and Human Subjects Protection

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 2019¹



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

¹1 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

Els Houtsmuller, PhD

Associate Director, HDDR

Amanda Barbeau, MPH

Program Associate, CEDS

Andrew Hu, MPP

Director, Engagement



Maternal Mortality and Morbidity (MMM)

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

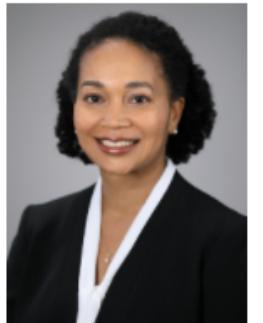
Blog Topics: [Executive Director's Blogs](#),
[Funding Awards](#),
[Research](#)

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*

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

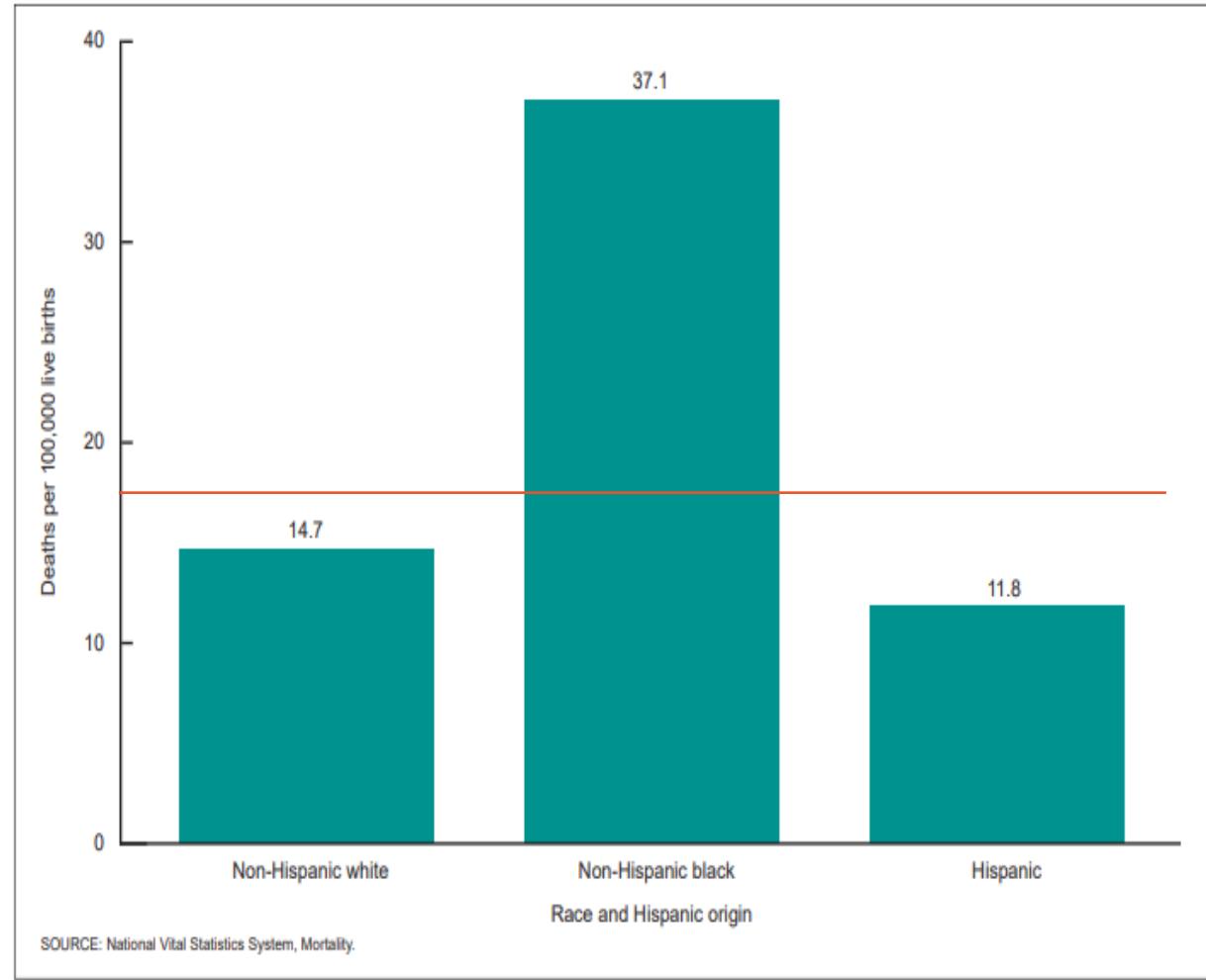
Please find additional details on the [PCORI website](#) and within the handout provided.

Maternal Mortality: US Rates and Disparities

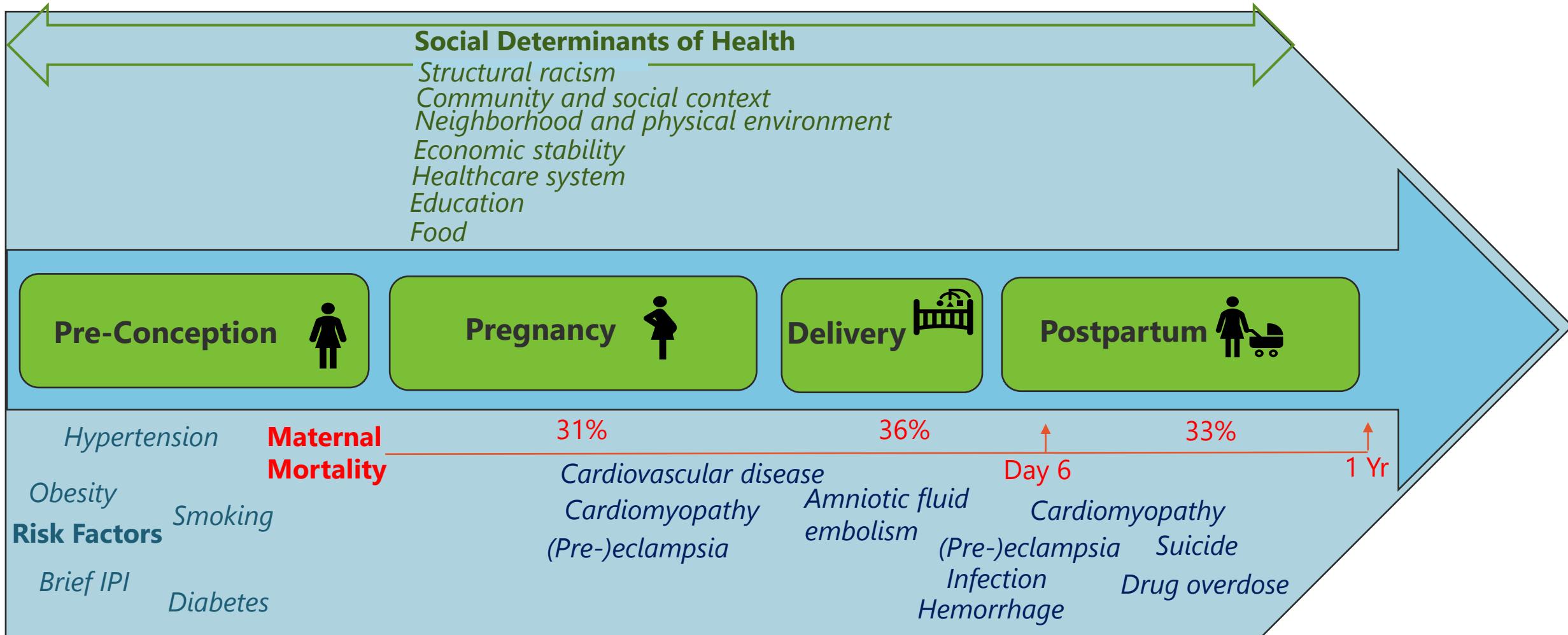


2017 Maternal Mortality Rates per 100,000 Live Births by Country

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 Framework: More than Just Pregnancy and Delivery



Maternal Mortality Framework: Opportunities for Healthcare Intervention



Intervention Opportunities

Addressing risk factors
Pregnancy planning
Mental health
Social needs

Wellness maintenance
(physical, mental health)
Complications
Social needs

Labor interventions
(induction, c-section)
Complications

Pregnancy spacing
Complications
Mental health
Social needs

Pre-Conception



Pregnancy



Delivery



Postpartum



Hypertension
Obesity
Risk Factors
Smoking
Brief IPI
Diabetes

Maternal Mortality

31%

36%

Day 6

33%

1 Yr

60% deaths preventable

Maternal Mortality

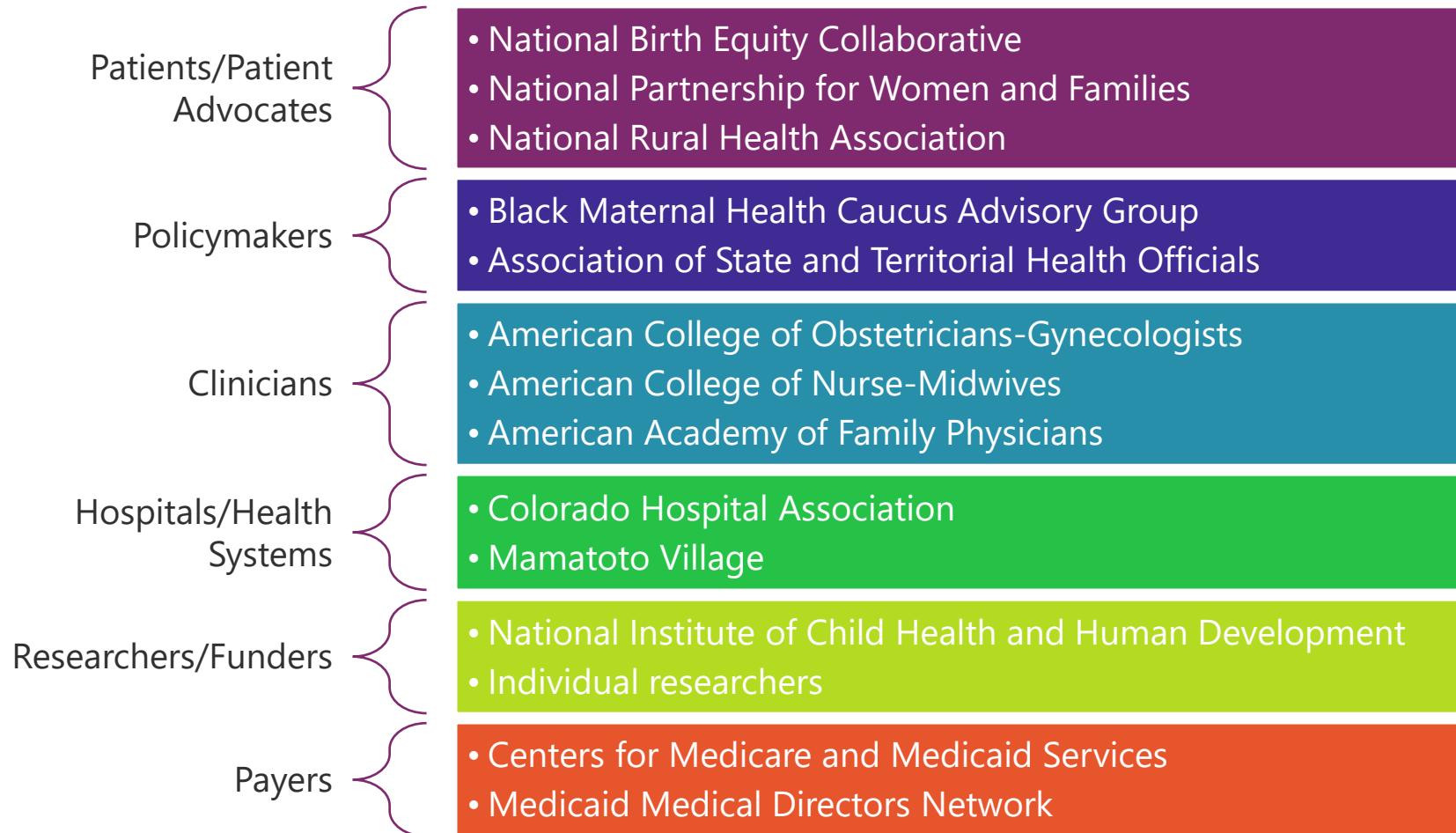
Stakeholder Engagement to Date: A Sample



- 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)

Amanda Barbeau, CEDS



Intellectual and Developmental Disabilities



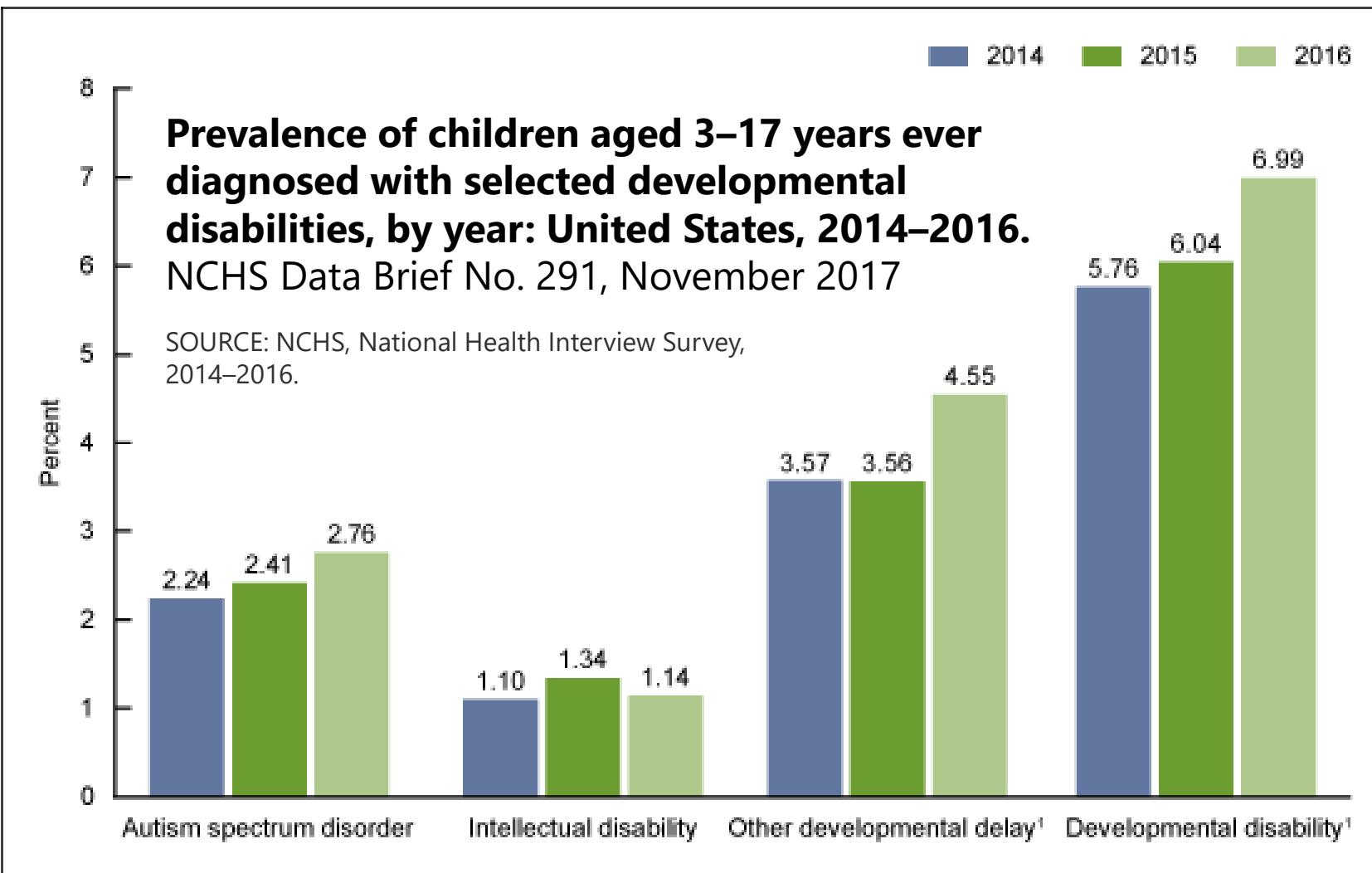
Intellectual and developmental disabilities (IDD) are disorders that are usually present at birth and that negatively affect the trajectory of the individual's physical, intellectual, and/or emotional development. [NICHD]

- **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]

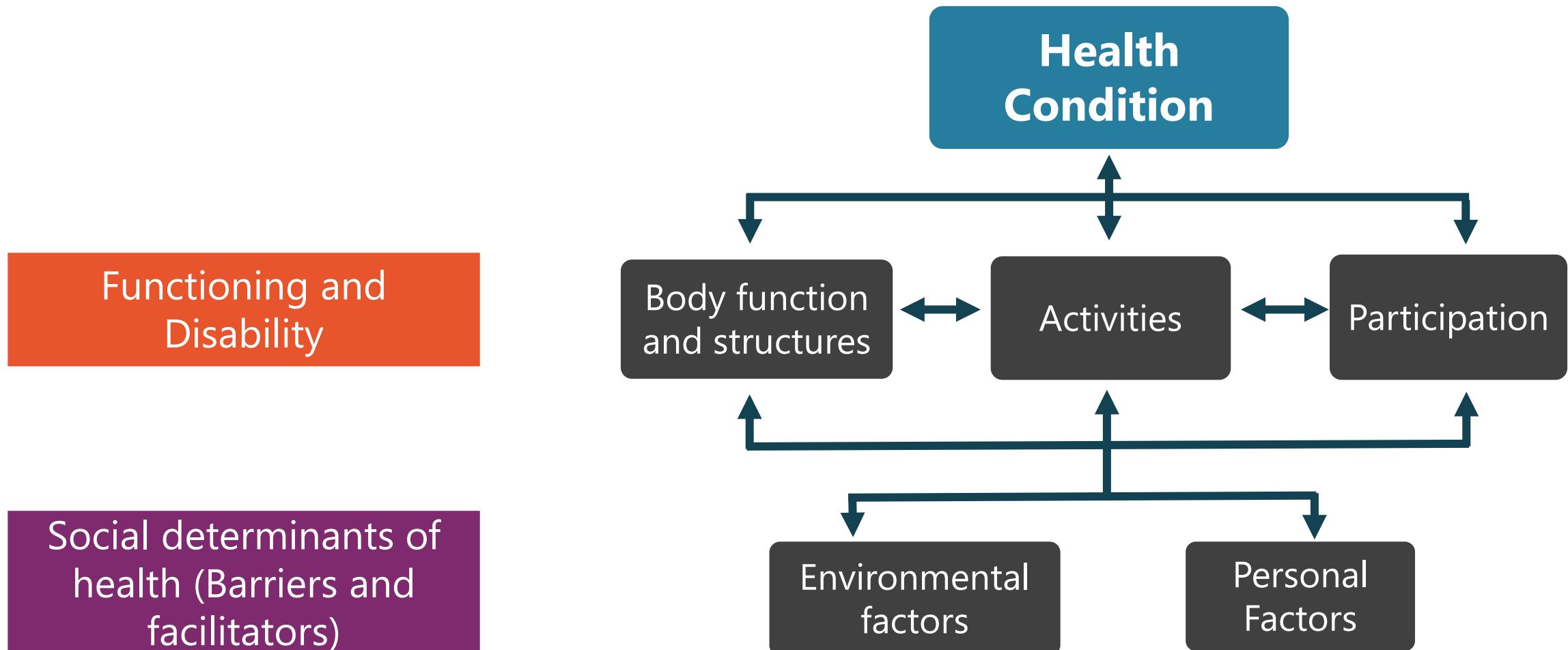
Not all developmental disabilities include limitations in cognitive ability.

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.

pediatric



adolescent



adult

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

Thank you!

Cost Provision

Andrew Hu, Engagement



Overview of New Statutory Authority

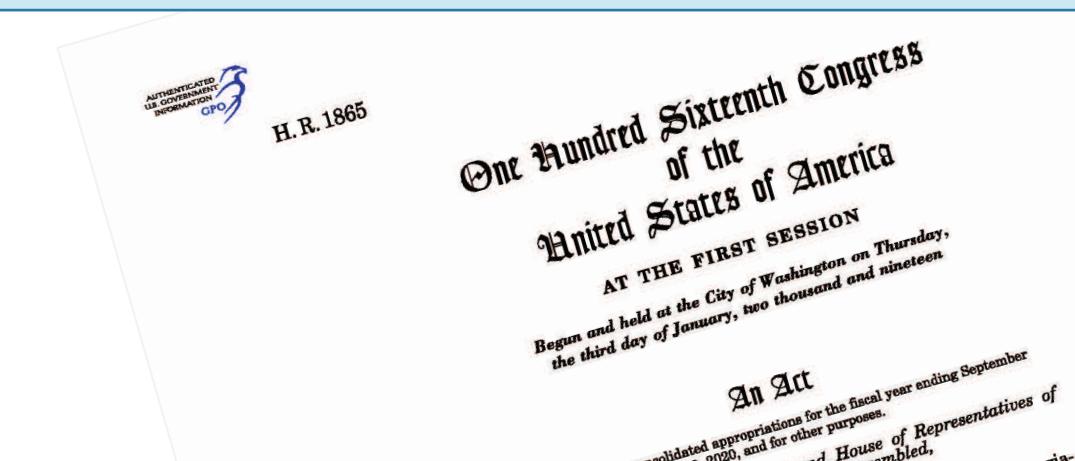


PCORI's reauthorizing legislation directs PCORI to capture, as appropriate, the full range of outcomes data in the course of our research studies.

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

- PCORI Board of Governors approved the release of the proposed principles for public comment on **September 14, 2020**

Seeking Public Input

- 60-day public comment period
- Webinar series
- Advisory panels

Revising Principles & Guidance

- Revise principles based on public input
- Final approval of principles in February or March 2021
- Guidance to applicants in PCORI Funding Announcements in Spring 2021



WE ARE HERE

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 \(Public Comment Webform\)](#)

The screenshot shows the PCORI website with a blue header bar containing links for BLOG, NEWSROOM, FIND IT FAST, HELP CENTER, SUBSCRIBE, CAREERS, and CONTACT US. Below the header is a navigation bar with links for ABOUT US, RESEARCH & RESULTS, TOPICS, ENGAGEMENT, FUNDING OPPORTUNITIES, and MEETINGS & EVENTS. The main content area features a breadcrumb navigation: Engagement > Engage with Us > Provide Input > Proposed Principles for the Consideration of the Full Range of Outcomes Data (2020). Below this, there is a section titled "Proposed Principles for the Consideration of the Full Range of Outcomes Data (2020)". It includes a paragraph about the Board of Governors' approval, a list of principles, and a "View Proposed Principles and Submit Comments" button. At the bottom, there is a "Related Webinars" section with links to two events: "Considering the Full Range of Outcomes in PCORI Research – Patients, Caregivers and Consumers" and "Considering the Full Range of Outcomes in PCORI Research – Payers, Purchasers, Providers, Health Systems and Pharmaceutical Industry".

The screenshot shows the PCORI website with a blue header bar containing links for BLOG, NEWSROOM, FIND IT FAST, HELP CENTER, SUBSCRIBE, CAREERS, and CONTACT US. Below the header is a navigation bar with links for ABOUT US, RESEARCH & RESULTS, TOPICS, ENGAGEMENT, FUNDING OPPORTUNITIES, and MEETINGS & EVENTS. The main content area features a title "Proposed Principles for the Consideration of the Full Range of Outcomes Data (2020)". It includes a paragraph about the Board of Governors' approval, a list of principles, and a "View the Proposed Principles for Consideration (pdf)" button. Below this, there is a "Submit Comments" section with a text input field. At the bottom, there is a "Principle 1: PCORI-funded research may consider the full range of outcomes important to patients and caregivers, including burdens and economic impacts." section with a detailed description of the principle.

Closing Remarks/Adjourn

Cornell Wright, MPP

CEDS Chair

Lawrence Goldberg, MD

CEDS Co-Chair



Thank you!

