

# Clinical Effectiveness and Decision Science

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PCORI Virtual Advisory Panel Meeting

May 20, 2022 | 1:15–5 pm (ET)



# Welcome & Introductions

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**Kim Bailey, MSc**

Associate Director,

Clinical Effectiveness and Decision Science (CEDS)

**Julie Eller**

CEDS Advisory Panel Co-Chair



# Housekeeping

- Today's webinar is open to the public and is **being recorded**.
  - 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 Spring 2022 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



- **Julie Eller, BS, MPH Candidate**  
CEDS Advisory Panel Co-Chair
- **Holly Ramsawh, PhD**  
Senior Program Officer, Clinical Effectiveness and Decision Science
- **Julia Song, MPH**  
Senior Program Associate, Clinical Effectiveness and Decision Science

# CEDS Panel Co-Chair

## Julie Eller, MPH Candidate

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



# Agenda

Time	Duration	Activity
1:15 pm	15 min	Welcome and Introductions
1:30 pm	20 min	CEDS Program Update
1:50 pm	60 min	Intellectual and Developmental Disabilities
2:50 pm	10 min	Break
3:00 pm	60 min	Update on Strategic Planning
4:00 pm	45 min	Healthcare Cost and Value
4:45 pm	15 min	Closing Remarks/Adjourn

# **CEDS Advisory Panel**

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Fall 2022 - Fall 2023  
Members





# CEDS Panel Members 2021



## **Patients, Caregiver Advocates, and Advocacy Organizations**

- Helen M. Beady, EdD, MEd
- Julie Eller, BS
- Christina Nyquist, BA
- Jennifer Olsen, DrPH
- Michael Philbin, PhD
- Sarah Wang, BS

## **Payers, Industry, and Policy Makers**

- Adjoa Adofo Kyerematen, MS
- Danielle Bargo, MSc
- Eric Cannon, PharmD, FAMCP
- David Webster, MD, MBA

## **Clinicians and Researchers**

- William Bennett, MD, MS
- Kari Gali, DNP, APRN, PNP-BC
- Karen Giuliano, PhD, MBA, RN
- Lisa Goldman Rosas, PhD, MPH
- Timothy Gong, MD, FACC
- Samantha Harden, PhD, RYT® 500
- Michael Hoerger, PhD
- Susan Johnson, MBA, MS
- Joey Mattingly, PharmD, PhD, MBA
- Rick Rader, MD

# CEDS Program Update

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**Holly Ramsawh, PhD**

Senior Program Officer,  
Clinical Effectiveness and Decision Science

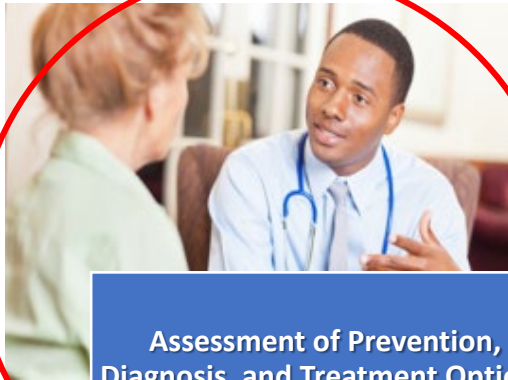


# Clinical Effectiveness and Decision Science



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



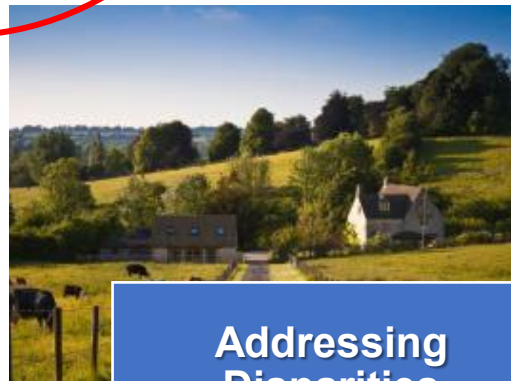
Assessment of Prevention,  
Diagnosis, and Treatment Options



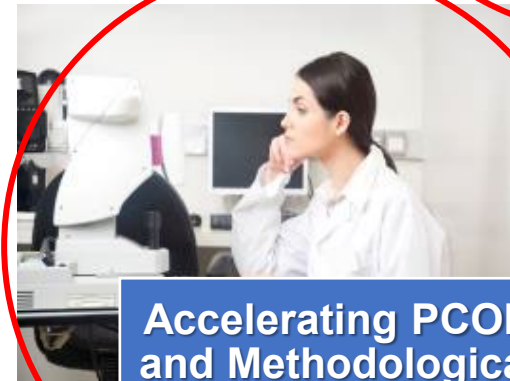
Improving  
Healthcare Systems



Communication &  
Dissemination  
Research



Addressing  
Disparities



Accelerating PCOR  
and Methodological  
Research

# Research Awards: Current CEDS Portfolio



PCORI HAS AWARDED OVER  
**\$1.16** **BILLION** **387**  
TO FUND  
COMPARATIVE EFFECTIVENESS STUDIES IN  
CLINICAL EFFECTIVENESS AND DECISION SCIENCE

As of March 2022

Funding Mechanism	# of Projects
Broad	318
Pragmatic	24
Targeted	45

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

# New Research Awards

A green circle with a dark green border containing the text "Recent Awards" in a bold, dark green font.

## Recent Awards

4

**Suicide** Prevention: Brief Interventions for Youth  
Targeted PFA Awards

3

Nonsurgical Options for Women with Urinary  
Incontinence Targeted PFA

2

Improving Postpartum Maternal Outcomes for  
Populations Experiencing Disparities Targeted PFA

1

Pragmatic Clinical Studies PFA

# New Research Awards: A Sampling

Study Title	Site	Cycle 2 2021 PCORI Funding Announcement (PFA)
Efficacy of pharmacologic management of ADHD in children and youth with autism spectrum disorder	The Research Institute at Nationwide Children's Hospital	PCS
Tailoring incontinence care: onabotulinumtoxinA for urgency urinary incontinence among older women	Dartmouth Geisel School of Medicine	Nonsurgical Options for Women with Urinary Incontinence

# Currently in Review: Cycle 3 2021



## Awards will be announced July 2022

- Broad
  - Special Areas of Emphasis:
    - Telehealth for Chronic Disease Management among Vulnerable Populations with Complex Needs
    - Addressing Systemic Racism, Discrimination, and Bias in Healthcare Systems and Care Delivery
- Phase Large Awards for Comparative Effectiveness Research
- Brief Interventions for Adolescent Alcohol Use
- Comparative Effectiveness of Multimodal Interventions to Prevent Osteoporotic Fractures
- Healthy Aging: Optimizing Physical and Mental Functioning Across the Aging Continuum
- Comparative Effectiveness of Novel Pharmacologic and Evidence-Based Nonpharmacologic Treatments for Migraine Prevention



# Currently in Review: Cycle 1 2022



## Awards will be announced December 2022

- Broad Pragmatic Studies
  - Special Areas of Emphasis:
    - Addressing Racism, Discrimination, and Bias in Healthcare Systems and Care Delivery
    - Health System-Level Interventions to Improve Healthcare Worker Well-Being and Reduce Burnout
- Brief Interventions for Adolescent Alcohol Use
- Comparative Effectiveness of Interventions Targeting Mental Health in Individuals with Intellectual and Developmental Disabilities
- Prevention, Early Identification, and Treatment of Delirium in Older Adults

# Currently Open: Cycle 2 2022



## Awards will be announced March 2023

- Broad Pragmatic Studies
  - Special Areas of Emphasis:
    - Addressing Racism, Discrimination, and Bias in Healthcare Systems and Care Delivery
    - Aspirin to Prevent Preeclampsia\*
    - Caregiver-Delivered Interventions for Intellectual and Developmental Disabilities
    - Optimizing Prevention and Treatment of Postpartum Hemorrhage\*
- Improving Postpartum Maternal Outcomes for Populations Experiencing Disparities
- Health System Strategies to Address Disparities in Hypertension Management & Control

# Upcoming: Cycle 3 2022



**Funding announcement will open on September 7, 2022**

**Awards will be announced July 2023**

- Phased Large Awards for Comparative Effectiveness Research
- Broad Pragmatic Studies

# Intellectual and Developmental Disabilities

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**Amanda Barbeau, MPH**, Program Officer

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

Clinical Effectiveness and Decision Science



# Origins of IDD Prioritization

2019 – Congress reauthorizes PCORI – Amending legislation to specify two new national priority areas:

Maternal mortality

Intellectual and developmental disabilities



Implementation of mandate will include:

A **long-term commitment** to priority areas of investment

Ongoing opportunities for engagement to identify stakeholder driven topics and patient-centered outcomes



This means IDD is set as a national priority area by law, thus a long-term priority area of investment through broad funding announcements, special areas of emphasis funding announcements, and topic specific funding announcements

# Long Term Investment **IDD as a Research Priority**

- PCORI has a dedicated IDD Workgroup that was created to guide PCORI's efforts in meeting the legislative mandate of IDD as a research priority.
- Over the ensuing years, this Workgroup will partner with stakeholders to develop and implement a research agenda that aims to provide evidence to help neurodiverse individuals get the best care for them.



# Funding High Priority Research:

*Focused funding to address the needs of individuals with IDD*



## Previous/Closed PCORI Funding Announcements

- Special Area of Emphasis: *Improving Care for Individuals with Intellectual and/or Developmental Disabilities Growing into Adulthood* – Cycle 3 2020; Cycle 1 2021; Cycle 2 2021
- tPFA: *Comparative Effectiveness of Interventions Targeting Mental Health Conditions in Individuals with Intellectual and Developmental Disabilities* – Cycle 2 2021

## Open PCORI Funding Announcements

- tPFA: [\*Comparative Effectiveness of Interventions Targeting Mental Health Conditions in Individuals with IDD\*](#) – Cycle 1 2022 (revised/reissued from Cycle 2 2021)
- Special Area of Emphasis: [\*Caregiver-Delivered Interventions for Intellectual and Developmental Disabilities\*](#) – Cycle 2 2022

## Upcoming PCORI Funding Announcements

- Future funding mechanisms and topics TBD for Cycle 3 2022 and 2023 cycles

**Note: PCORI is always seeking investigator-initiated research proposals through all research priority areas in the Broad Pragmatic Studies funding mechanism.**

# Topic: Sleep Disturbance in Individuals with IDD

- Background
  - Insomnia and other sleep complaints are common in the general population.
  - Many sleep disturbances are treatable, with behavioral interventions as a first-line treatment.
  - Among those with IDD, sleep disturbances are prevalent, but there are few validated interventions.
- CER needs
  - Direct comparison of pharmacologic and non-pharmacologic interventions
  - Comparison of concurrent or sequential treatment regimens
  - Treatment delivery modes (e.g., caregiver-delivered, mobile apps, telehealth)
  - Dose and duration, longer-term follow-up



# Topic Scoping:

## Sleep disturbance in individuals with IDD

- Discussion
  - What are the decisional dilemmas people with IDD, their caregivers, and their clinicians face that could be addressed through comparative effectiveness research (CER)? What are the important CER questions?
  - Are there specific groups or conditions that should be of focus, or should the focus be broad?
  - What are the most meaningful and relevant outcomes to consider?
  - Given few experts in both IDD and sleep disturbance, how do we maximize the likelihood of strong applications and ensure a robust review process?

# Topic Prioritization:

## Potential future topics for consideration and ranking



- **Potential future topics to rank:**
  - Dental health
  - Physical health
  - Assistive/augmentative technologies
  - COVID and telehealth
  - Maternal morbidity and mortality in IDD
  - Transition to adulthood

**Panelists:** Go to [www.menti.com](https://www.menti.com) and enter the code provided in the chat window.

# Open Discussion:

## Additional IDD topics for CER?



- Are there additional topics that may be important to consider for future IDD CER funding initiatives?

# Contact IDD Workgroup

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IDDPCORI@PCORI.org



**BREAK**



10 min

Please return at 3 pm (ET)

# Update on Strategic Planning: PCORI's Strategic Plan

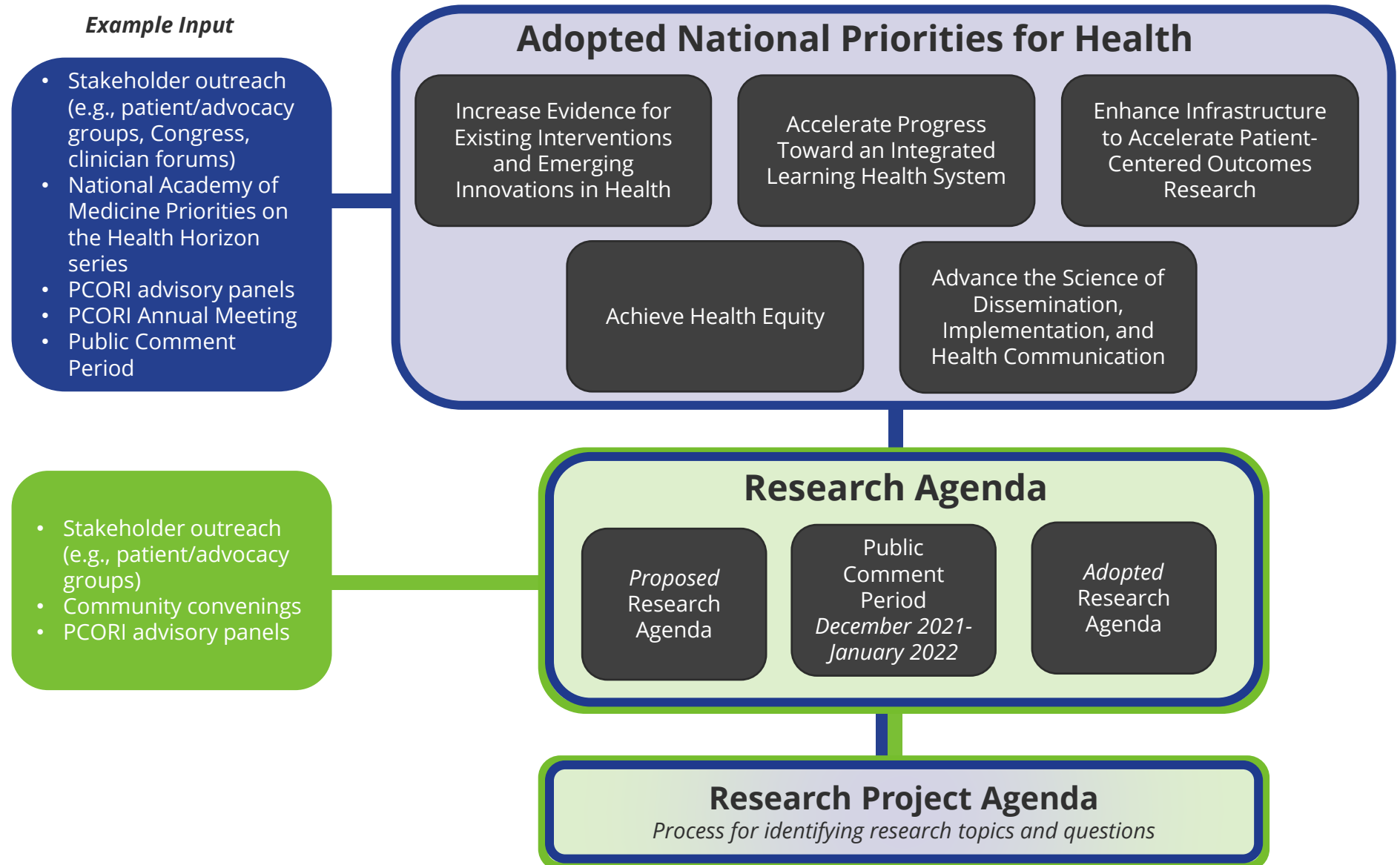
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**Laura Lyman Rodriguez, PhD**

Deputy Executive Director for  
Strategy and Planning



# Stakeholder and Public Input Inform Work



# Adopted National Priorities for Health



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

**Goal:** 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.



## Enhance Infrastructure to Accelerate Patient-Centered Outcomes Research

**Goal:** 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.



## Advance the Science of Dissemination, Implementation, and Health Communication

**Goal:** Advance the scientific evidence for and the practice of dissemination, implementation, and health communication to accelerate the effective sharing of comparative clinical effectiveness research results for public understanding and uptake into practice.



## Achieve Health Equity

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



## Accelerate Progress Toward an Integrated Learning Health System

**Goal:** 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 understands and serves the needs and preferences of individuals.



# When We Last Spoke

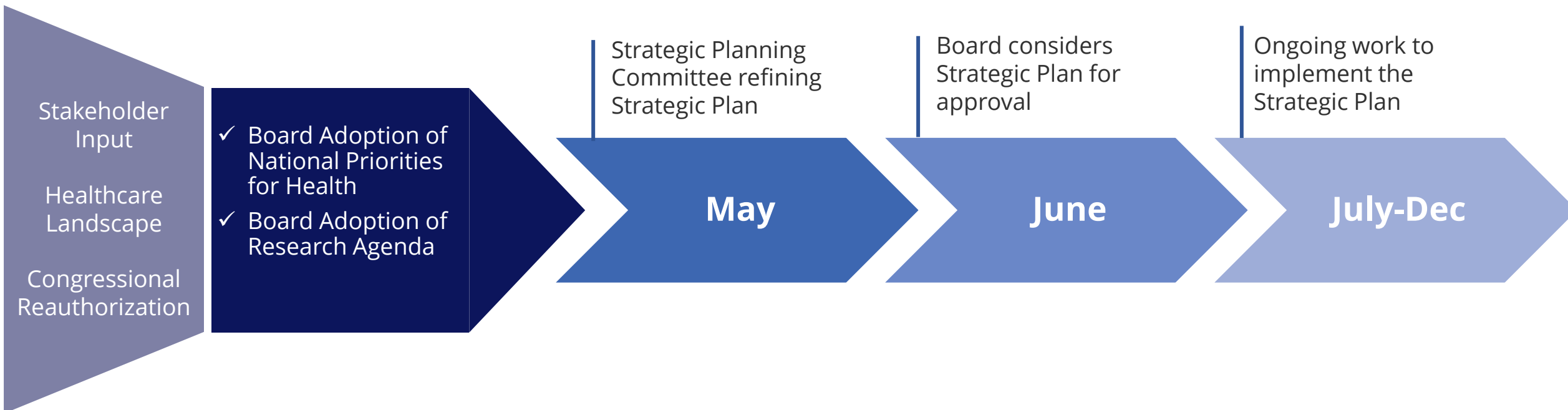
- This panel considered the proposed Research Agenda and how it could support a research portfolio to meet current and future needs.
- Panel members shared insights regarding PCORI's future work including the following:
  - The opportunity for the Research Agenda to support collaborative, multi-disciplinary research teams (e.g., specialties and disciplines across the care continuum)
  - The opportunity for the Research Agenda to support cross-cutting areas for study (e.g., promotion and practice of shared decision-making)
- This was integrated with input from other convenings, meetings, and discussions.

# Adopted Research Agenda

Fund comparative clinical effectiveness research that

- ▶ Fills patient- and stakeholder-prioritized evidence gaps and is representative and inclusive of diverse and underrepresented patient populations and settings
- ▶ Advances the achievement of health equity and elimination of disparities with an emphasis on overcoming the effects on health and healthcare outcomes of racism, discrimination, and bias
- ▶ Builds the evidence base for emerging interventions by leveraging the full range of data resources and partnerships
- ▶ Examines the diverse burdens and clinical and economic impacts important to patients and other stakeholders
- ▶ Focuses on health promotion and illness prevention by addressing health drivers that occur where people live, work, learn, and play
- ▶ Integrates implementation science and advances approaches for communicating evidence so the public can access, understand, and act on research findings

# Anticipated Timeline



# Developing a Comprehensive Approach

- Core Strategic Plan document
- Digital format (e.g., clickable, interactive webpages)
- Printable “at-a-glance” handouts (e.g., two-page PDFs)

## Accessibility



- Demonstrate how PCORI achieves its mission
- Opportunity for stakeholders to see themselves in PCORI’s vision
- Serve as a communication tool
- Serve as the basis for PCORI’s next phase

## Purpose



- Patients and caregivers
- Researchers and clinicians
- Congress and GAO
- Healthcare purchasers, payers, industry, hospitals, and other health systems
- Broader health community

## Audiences



# Outline of Substantive Components in Strategic Plan



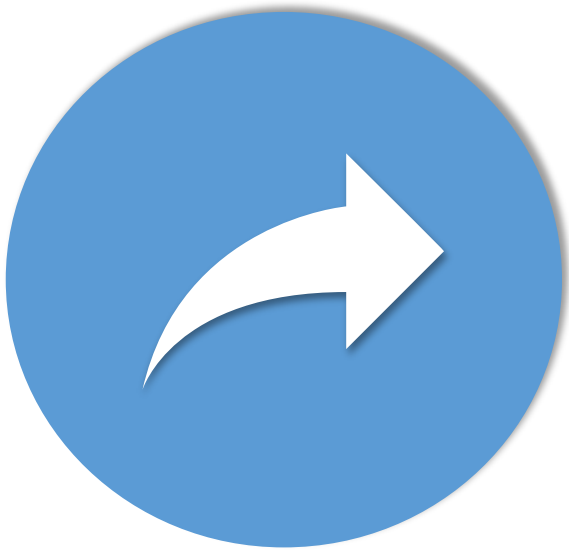
- **Introduction and A Bold Vision for PCORI's Second Decade**
  - Shares, briefly, PCORI's history and how we envision continuing our work such as the continued importance of diversity, equity, and inclusion and being responsive to an evolving health care and research landscape
- **A Holistic Approach to Generating and Disseminating Patient-Centered CER**
  - Describes comprehensive set of activities (e.g., funding CER, engagement, D&I, infrastructure) that flow from the National Priorities for Health and facilitate the generation and dissemination of evidence to improve clinical decision making
  - Includes the National Priorities for Health and Research Agenda
- **Measuring Success and Monitoring Progress**
  - Provides general approach for assessing PCORI's progress towards the National Priorities for Health

# Moving Towards Implementation Planning

- We are excited to *begin discussions* with Advisory Panels related to implementation planning. To start, we would like your input on strategic opportunities related to the Research Project Agenda (RPA).
- We are particularly interested in hearing about promoting an effective, transparent process for developing the RPA. Currently, this is in a *transition* and we are considering opportunities to revise the process with an eye towards enhancing ability to be *nimble, adaptive, and responsive* to changes in the health environment.
- Aim for Research Project Agenda process that is:
  - Stakeholder-Driven
  - Responsive
  - Transparent
  - Systematic
  - Efficient



# Anticipated Next Steps



- Use feedback garnered from discussions to inform our process considerations along with actionable steps for implementation
- Share update on process development with this panel at a future meeting

# Beginning Discussions to Help Inform Implementation Planning

- What are meaningful ways to engage with stakeholder communities represented on this panel to solicit ideas and inform topic development?
  - How can we best ensure that engagement is inclusive of a diversity of stakeholder perspectives?
- What does a responsive, transparent Research Project Agenda development process look like from your perspective?
  - How can we best ensure that this process is inclusive and representative of a diversity of views?
- Given the broad, integrated nature of the adopted National Priorities for Health and Research Agenda, how could PCORI demonstrate relevance to stakeholder communities represented by this panel?
- Based on this panel's expertise in comparative effectiveness research, what are this panel's suggestions to stay up to date on emerging issues in this space?





# Healthcare Cost and Value

## Addressing the Full Range of Outcomes

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**Greg Martin, PhD**

Acting Chief Engagement and Dissemination Officer,  
Engagement

**Olga Khavjou/Kristen Giombi**

RTI



# Agenda



- Overview of PCORI Activity
  - Greg Martin, Acting Chief Engagement and Dissemination Officer
- Current Learnings
  - Olga Khavjou/Kristen Giombi, RTI
- Discussion

# Overview

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## PCORI Approach to Healthcare Cost and Value: Framework for Activities

### Collection of the Full Range of Outcomes

PCORI can ensure the appropriate and relevant data are identified and collected in PCORI awards

Support broadening of PCORI guidance to awardees through literature reviews, patient & stakeholder focus groups to clarify types of potential burdens, costs, and economic impacts to be included in PCORI-funded research, and data sources

Develop guidance for capture of specific types of potential burdens or costs consistent with a patient-centered perspective, as a basis for PCORI guidance to awardees and the field

### Informing the Value Conversation

PCORI can help advance patient-centered approaches to the value conversation by supporting understanding of stakeholder perspectives on what constitutes “patient-centered value”

Conduct a landscape review of current stakeholder statements, perspectives, and frameworks around value

Convene patient and stakeholder groups to identify the components or attributes necessary in “patient-centered value”

### Supporting Policy Priorities

PCORI can consider undertaking specific activities that contribute to priority policy goals (i.e., generating evidence to inform healthcare value discussions)

Consider placing an emphasis on research focused on emerging technologies/therapies

Consider building upon evidence synthesis capabilities to provide more timely information

# Update on Activities and Deliverables

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## Landscape Review

- How are different stakeholder communities defining “patient-centered value”?
- Where are the similarities and where are the differences?

# Value Attributes

Category	Attribute	Patients/ caregivers	Clinicians	Policymakers	Payers	Purchasers	Life Sciences Industry	Hospital/ Health System	Researchers/ Funders	Number of Stakeholders
Life and social impact	Emotional/mental status/well being									2
	Fatigue									2
	Physical abilities/well being/independence									2
	Insurance value (physical and financial risk)									1
	Future planning									2
	Fear of rejection by family/society									1
	Support network									2
	Relationship with family/peers									1
	Participation in social activities									1
	Social history (cultural barriers and religious beliefs)									2
		36	22	7	10	10	5	20	31	

## Identifying the Components of Patient-Centered Value

- What does patient-centered value mean to different communities?
- What elements, attributes, or components do different stakeholders consider to be part of value measurement?



# Discussion



# Questions for the Panel

- Do the direction, activities, and goals resonate with you?
- Will the activities meet the informational needs of your stakeholder community?
- What additional considerations should be kept in mind as we proceed?

# Closing Remarks

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**Julie Eller**  
CEDS Co-Chair



**Thank you!**

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