

# Advisory Panel on Clinical Trials Spring 2022 Meeting

May 13th, 2022

1:00 PM – 5:00 PM ET

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United States (Toll-free): 1 866 901 6455

Access code: 111-801-423

Webinar URL: <https://attendee.gotowebinar.com/register/356996454303191312>

# Housekeeping

- Meeting is available to the public and is being recorded.
- Members of the public are invited to listen in, and the recording will be made publicly available after the event on PCORI's website.

# GoToWebinar Housekeeping



- Remember to mute your microphone if you are not speaking.
- If possible, please turn on your video camera when speaking.
- If you have poor connection, make sure to dial in through phone (not computer).
- If you are experiencing technical difficulties, contact “PCORI AV” via the chat function.
- To queue to speak, submit your name through the chat to “Organizers Only”.
  - Feel free to use your camera when speaking.
- If you would like to send a comment to the entire group, select “All Attendees” in the chat function.

# COI Statement



Welcome to the CTAP Spring 2022 Virtual Meeting. I want to remind everyone that disclosures of conflicts of interest of members of CTAP are publicly available on PCORI's website and are required to be updated annually. Members of the CTAP are also reminded to update your conflict of interest disclosures if the information has changed. You can do this by contacting your staff representative, Megan Leimkuhler.

If the CTAP will deliberate or take action on a matter 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, Megan Leimkuhler.

# Welcome and Goals for the Day

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**Michael Jones, PhD, MBA, RN (Chair)**

Richards Endowed Graduate Nursing Chair and Research  
Development Coordinator,  
Brenau University

**Anne Trontell, MD, MPH**

Associate Director,  
Clinical Effectiveness and Decision Science, PCORI

# PCORI Advisory Panels - Spring Sessions



PCORI advisory panels will meet between May 11 and May 20, with all panels having sessions on PCORI's strategic plan and healthcare cost and value. Links to the agendas and registration are below. All meetings will be virtual, schedule:

- May 11—[Advisory Panel on Healthcare Delivery and Disparities](#)
- May 13—[Advisory Panel on Clinical Trials](#)
- May 16—[Advisory Panel on Patient Engagement](#)
- May 18—[Advisory Panel on Rare Disease](#)
- May 20—[Advisory Panel of Clinical Effectiveness and Decision Science](#)

# Today's Agenda



Friday, May 13th		
Time	Agenda Item	Presenter
1:00	Opening welcome, logistics, COI, etc	Megan Leimkuhler
1:05	Welcome and Goals for the Day	Michael Jones, Anne Trontell
1:10	Executive Director Welcome	Nakela Cook
1:30	Strategic Planning	Laura Lyman Rodriguez
2:00	Health Care Cost and Value	Greg Martin, Olga Khavjou, Kristen Giombi
	Break (15 minutes)	
3:00	Advancing Health Equity	Sarah Ruiz, Vivian Towe
4:15	Panelist Suggestions for Future Meeting Topics	Anne Trontell
4:45	Closing and Thank You to Panelists	Michael Jones, Anne Trontell

# Executive Director Welcome

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





# Welcome PCORI's Executive Director



# CTAP Member Introduction



After your name is called, please introduce yourself briefly with your name, position, institution, and stakeholder role on CTAP

- Yuelin Li
- Jacqueline Corrigan-Curay
- Wenora Johnson
- Maryam Tabatabai
- Rowena Dolor
- Susan Lin
- Ilana Gareen
- Jennifer Christian
- Emily Largent
- Angelica Geter
- Elizabeth Turner
- Cristina Murray-Krezan
- Cindy Girman (*Ex Officio, MC*)

Attendance uncertain: Larry Kessler, Bill Tierney, Steve Goodman (*Ex Officio, MC*)

# Executive Director Remarks



**Nakela L. Cook, MD, MPH**, is PCORI's executive director. She is a cardiologist and health services researcher with a distinguished career leading key scientific initiatives engaging patients, clinicians, and other healthcare stakeholders.

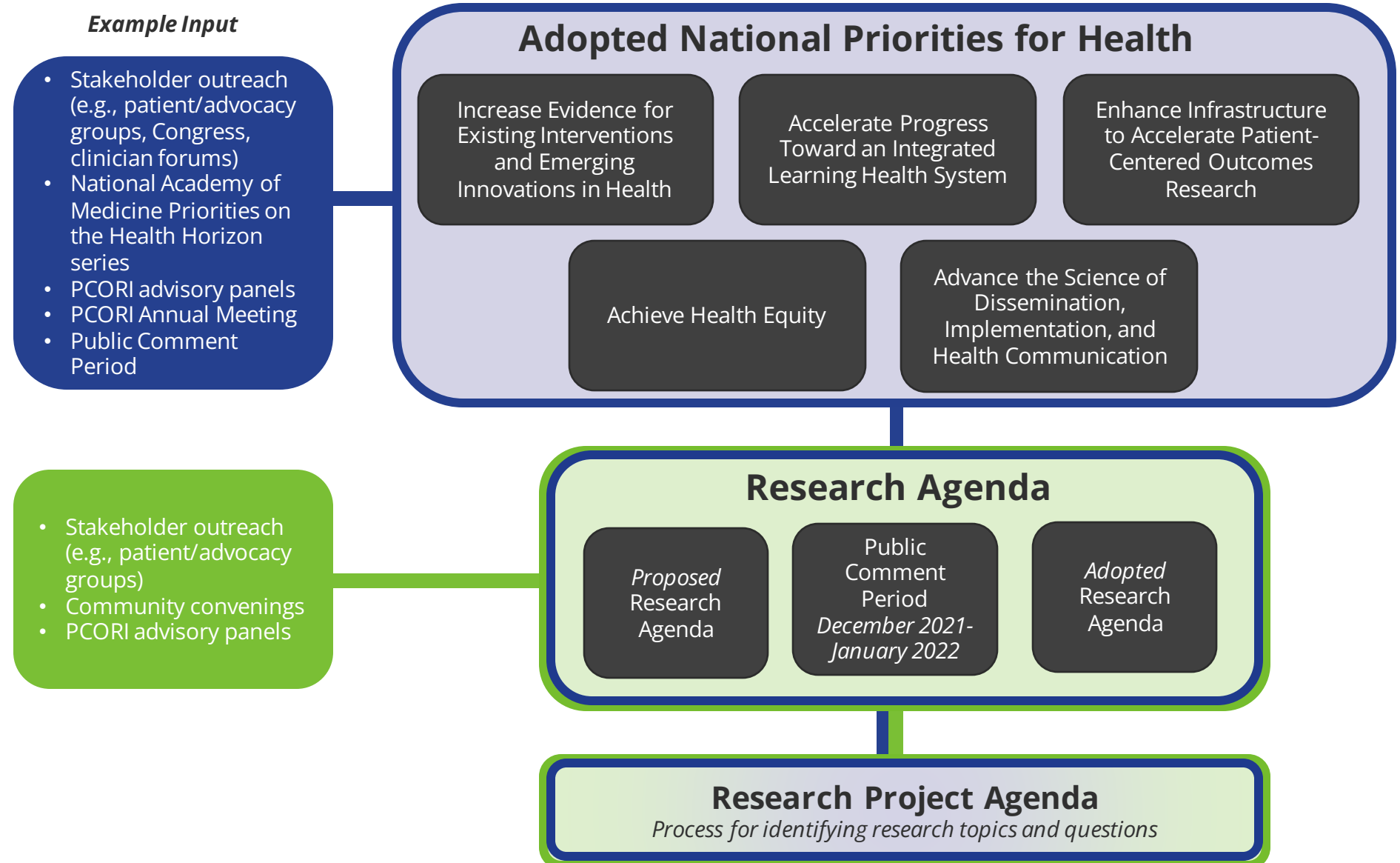
# Update on Strategic Planning: PCORI's Strategic Plan

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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, particularly best practices for achieving diverse, representative, and inclusive patient populations in clinical trials for patient-centered outcomes research.
- Panel members shared insights regarding PCORI's future work including the importance of the following:
  - Engaging communities and community partners early and often to build trust and partnerships.
  - Leveraging innovative approaches developed during the pandemic to recruit diverse populations (e.g., telehealth, digital messaging, online platforms).
  - Developing study materials that are easy to understand and are culturally appropriate for the population of interest while using trusted messengers to help with dissemination efforts.
- This was integrated with input from other convenings, meetings, and discussions.

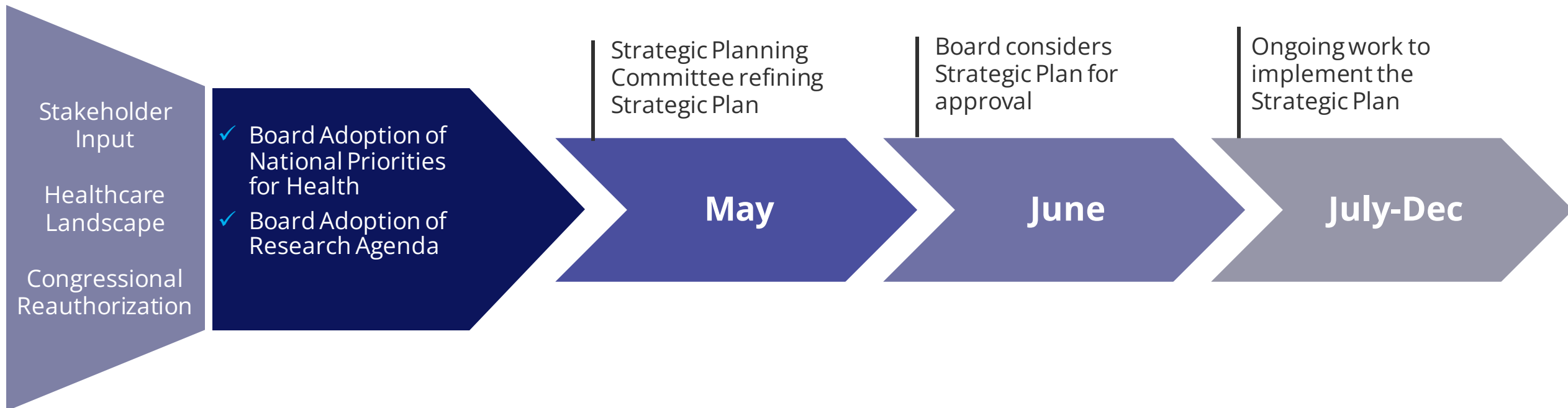
# Adopted Research Agenda

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



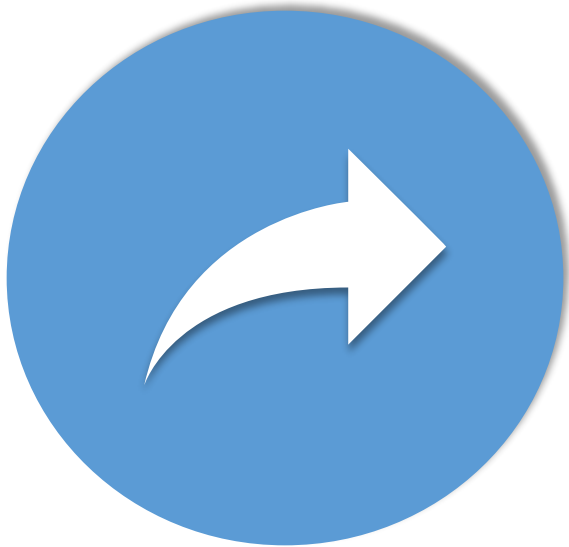
- **Introduction and A Bold Vision for PCORI's Second Decade**
  - Shares, briefly, PCORI's history and how we envision continuing our work such as the continued importance of diversity, equity, and inclusion and being responsive to an evolving health care and research landscape.
- **A Holistic Approach 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 (e.g., convenings, surveys, webinars)?
- What does a responsive, transparent Research Project Agenda development process look like from your perspective?
- 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 CTAP's expertise in clinical trials for PCOR, what are your suggestions to stay up to date on emerging issues in this space?



# Healthcare Cost and Value

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Addressing the Full Range of Outcomes

May 13, 2022

# Agenda

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# Agenda



- Overview of PCORI Activity
  - Greg Martin, Acting Chief Engagement and Dissemination Officer
- Current Learnings
  - Olga Khavjou, 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	Policy makers	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

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# Questions for the Panel

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

# **Break (15 min)**

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Feel free to turn off camera and mute  
your line.

Please remain logged into the webinar



# Advancing Health Equity in a Comparative Effectiveness Research Framework

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*Sarah Ruiz, PhD*

*Vivian Towe, PhD, MSc, MA*

May 13, 2022

# Purpose of today's presentation



- Highlight the specific intersections between Health Equity research and the PCORI's National Priorities for Health and Research Agenda
- Current and future PCORI efforts in Health Equity research
- Discussion questions

# Adopted National Priorities for Health

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- 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

# Advancing Health Equity in the Context of CER



- PCORI can encourage researchers to **submit proposals across the health research spectrum**, including addressing Social Determinants of Health (SDoH), evaluating health policies/programs, identifying approaches to improving healthcare access, and promoting disease prevention, while still maintaining its CER mission and ensuring that the improvement of health outcomes remains central.

# Entry Points for Comparative Effectiveness Research

**Upstream**

**Drivers of Health**

**Downstream**

## **Social Determinants**

- Housing
- Employment
- Education
- Income
- Racism/discrimination
- Environment

## **Preservation of health, promotion of well-being**

- Preventative services
- Screening
- Community resources for health
- Safe environment
- Green space

## **Behaviors**

- Smoking
- Nutrition
- Physical activity
- Violence
- Alcohol/drug use
- Sexual behavior

## **Institutional inequities**

- Government
- Legal
- School
- Financial
- Corporate

## **Healthcare system inequities**

- Access for the poorly served
- Systemic racism
- Provider bias
- Insurance coverage
- Payment models

## **Treatment/management of specific conditions/ care delivery**

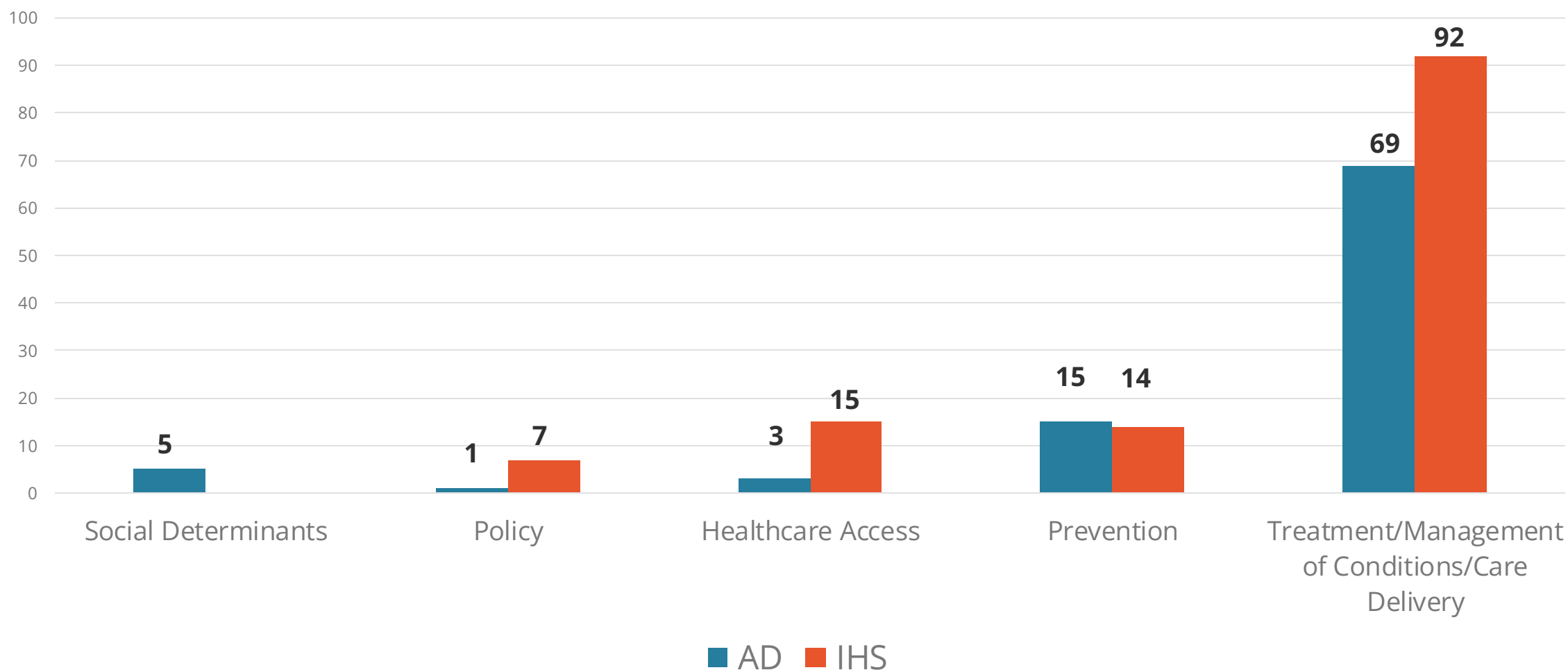
Policy, program, community, and systems-level interventions

Organizational and individual-level interventions



# Sample of studies across the CER spectrum

CER Entry Points



# Current funding opportunities to support *Achieve Health Equity*



- **Healthy Aging: Optimizing Physical and Mental Functioning Across the Aging Continuum (tPFA)**
  - Fund CER that aims to optimize the physical and mental functioning of community-dwelling older adults and their caregivers across the aging continuum
  - \$50m
- **Addressing Racism, Discrimination, and Bias in Healthcare Systems and Care Delivery (SAE)**
  - Fund CER on multicomponent, multilevel health system interventions addressing racism, discrimination, and bias based on race, ethnicity, and/or nativity.
  - Must include at least one institutional/organizational, community-based intervention, or educational and psychosocial intervention
  - \$20m

# Current funding opportunities to support *Achieve Health Equity*



- **Caregiver-Delivered Interventions for Intellectual and Developmental Disabilities (IDD) (SAE)**
  - Fund CER on evidence-based and/or commonly used caregiver-delivered interventions spanning the continuum of care for those with intellectual and/or developmental disabilities (IDDs)
  - \$20m

# Examples of “upstream” funded studies in PCORI’s portfolio



## Program and policy CER studies are typically natural experiments

- Comparative Effectiveness of Single-Site and Scattered-Site Permanent Supportive Housing on Patient-Centered and COVID-19 Related Outcomes for People Experiencing Homelessness
- Effect of High-Deductible Health Plans on Healthcare Use and Out-of-Pocket Costs for People with Bipolar Disorder
- Comparing Three Programs to Increase Access to Mental Health Care for Pregnant and Postpartum Patients

# Examples of recently funded “upstream” studies in PCORI’s portfolio



## Recent social determinant intervention studies are trials

- Vida Sana y Completa Trial: Treating obesity and food insecurity among Latina women
- Evaluating the Effectiveness of Mode of Meal Delivery on the Ability of Homebound Older Adults to Remain in the Community
- Patient-Centered Equity Enhancements to Reduce Disproportionate Unmet Mental Health Need of African American Children

# Discussion Questions

- What are topic areas of growth for health equity research using clinical trials?
- What are the potential challenges of testing social determinants interventions in trials?
- How might clinical trials consider optimizing recruitment to ensure diverse and underrepresented populations participate in health equity research?
- How can subgroup analyses (heterogeneity of treatment effects) in CER be leveraged to advance health equity?
- What would be fruitful topics to follow up with CTAP in the future?

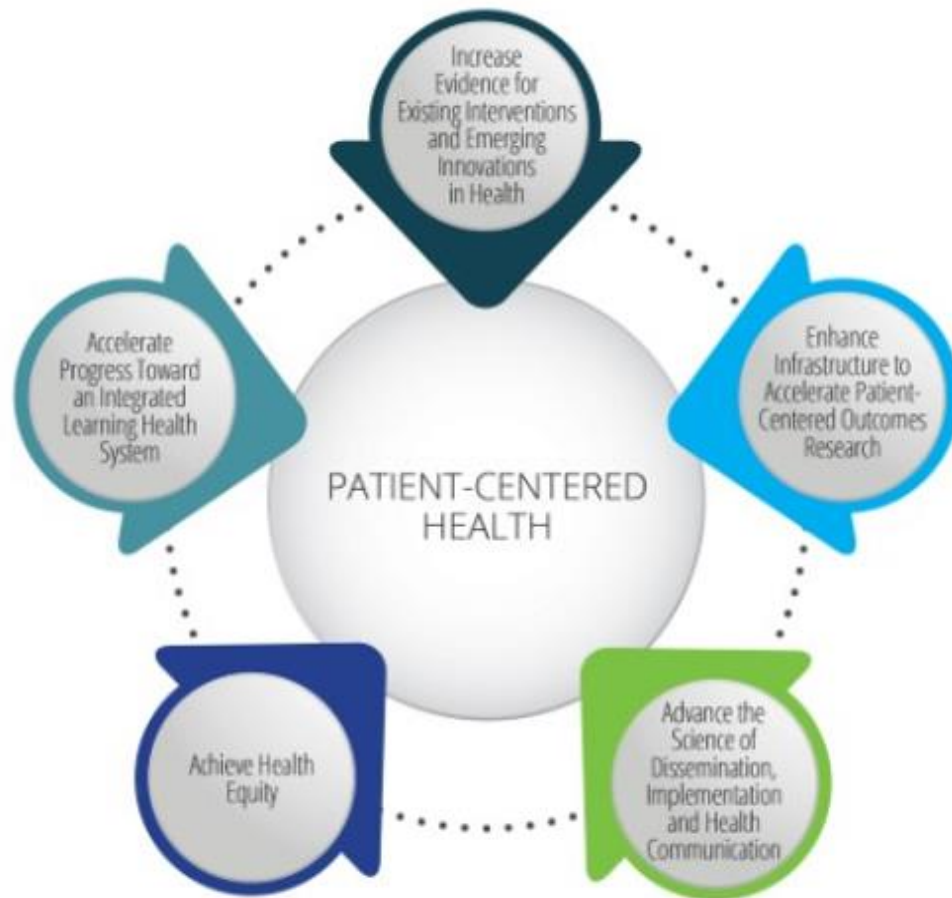
# Panelist Suggestions for Future CTAP Topics

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**Anne Trontell, MD, MPH**

Associate Director,  
Clinical Effectiveness and Decision Science, PCORI

# Proposed Research Agenda



-  Fund research that fills patient - and stakeholder - prioritized evidence gaps and is representative of diverse patient populations and settings
-  Fund research that aims to achievement of health equity and eliminate health and healthcare disparities
-  Fund research that builds the evidence base for emerging interventions by leveraging the full range of data resources and partnerships
-  Fund research that examines the diverse burdens and clinical and economic impacts important to patients and other stakeholders
-  Fund research that focuses on health promotion and illness prevention by addressing health drivers that occur where people live, work, learn, and play
-  Fund research that integrates implementation science and that advances approaches for communicating evidence so the public can access, understand, and act on research findings



# Potential Ideas for Future CTAP Discussion

- ❑ Further discussion of ways to enroll and retain diverse participants
- ❑ Interventions for individuals with IDD
- ❑ Trials of social and environmental drivers
- ❑ Hybrid effectiveness and implementation trials for complex interventions
- ❑ Decentralized trials using virtual/digital ways of conduct
- ❑ Trial efficiencies using EHRs, data linkages, other innovations
- ❑ Fostering Learning Health Systems using clinical trials
- ❑ Trials done in primary care and other less traditional settings such as public health institutions
- ❑ Collaboration with other PCORI Advisory Panels
  - Rare Disease Advisory Panel
  - Patient Engagement Advisory Panel (Science of Engagement)
- ❑ Emerging Practices in Clinical Trials
  - Comparison of irreversible comparisons in RCTs (surgical vs. nonsurgical trials, devices)
  - Sequential Multiple Assignment Randomized Trials (SMART) designs
  - Studies within a trial or add-on methodologic studies within clinical trials
- ❑ Phased Large Awards for Comparative Effectiveness Research (PLACER) Awards to add text

# Farewell to Panelists

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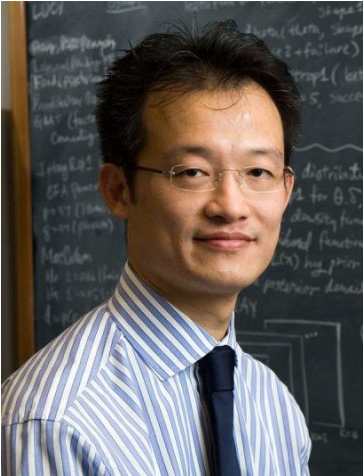
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# Farewell to Panelists



Yuelin Li



Wenora Johnson



Jacqueline Corrigan-Curay



Maryam Tabatabai

# Wrap Up and Next Steps

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**Michael Jones, PhD, MBA, RN (Chair)**

Richards Endowed Graduate Nursing Chair and Research Development  
Coordinator,  
Brenau University

**Anne Trontell, MD, MPH**

Associate Director,  
Clinical Effectiveness and Decision Science, PCORI

**Thank you!**

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