Board of Governors Meeting

via Teleconference/Webinar

September 14, 2020
1:30 PM – 4:45 PM

pcori
PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

Welcome and Introductions

Christine Goertz, DC, PhD

Chairperson, Board of Governors

Agenda

1:30 PM	**Call to Order and Welcome**
1:30-1:40	**Consent Agenda: Consider for Approval:** • Minutes of July 21, 2020 Board Meeting • Committee Leadership Nominations
1:40-2:15	**Consider for Approval:** • New Advisory Panelists, Chairs and Co-Chairs • Revised Methodology Committee and Governance Committee Charters • Cycle 1 2020 Dissemination and Implementation (D&I) Award Slate
2:15-3:15	**Executive Director's Report** • Highlights from the First 5 Months • COVID-19 Initiatives Update
3:30-4:30	**Consider for Approval for Posting for Public Comment:** • Cost Data Principles for Researchers
4:30-4:45	**Wrap-up and Adjournment**

Consent Agenda Items

Christine Goertz, DC, PhD

Chairperson, Board of Governors

Consent Agenda: Approval of Minutes and Appointments of Chairs, Vice Chairs, and Members for Committees

- That the Board approve:
  - Minutes from the July 21, 2020 Board meeting; and
  - the following nominations presented by the Governance Committee for the positions of Chair and Vice Chair of the specified Committees effective on September 23, 2020 for a two (2) year term, and the following nominations for Committee membership:

Chair, Vice Chair, and Committee Membership Nominations

- **Engagement, Dissemination, and Implementation Committee**
  - Mike Herndon – Chair
  - Sharon Levine – Vice Chair
- **Research Transformation Committee**
  - Kathleen Troeger – Chair
  - Kara Ayers – Vice Chair
- **Science Oversight Committee**
  - Alicia Fernandez – Chair
  - Christopher Friese – Vice Chair
- **Finance and Administration Committee**
  - Russell Howerton – Chair
  - Robert Zwolak – Vice Chair
  - Christine Goertz – Member
    - *Mike Herndon will no longer serve as a member of FAC*

Board Vote

Call for a Motion to:	• **Approve** each of the Motions on the Consent Agenda
Call for the Motion to be Seconded:	• **Second** the Motion • If further discussion, may propose an **Amendment** to the Motion or an **Alternative** Motion
Voice Vote:	• Vote to **Approve** the **Final** Motion • Ask for votes in favor, opposed, and abstentions

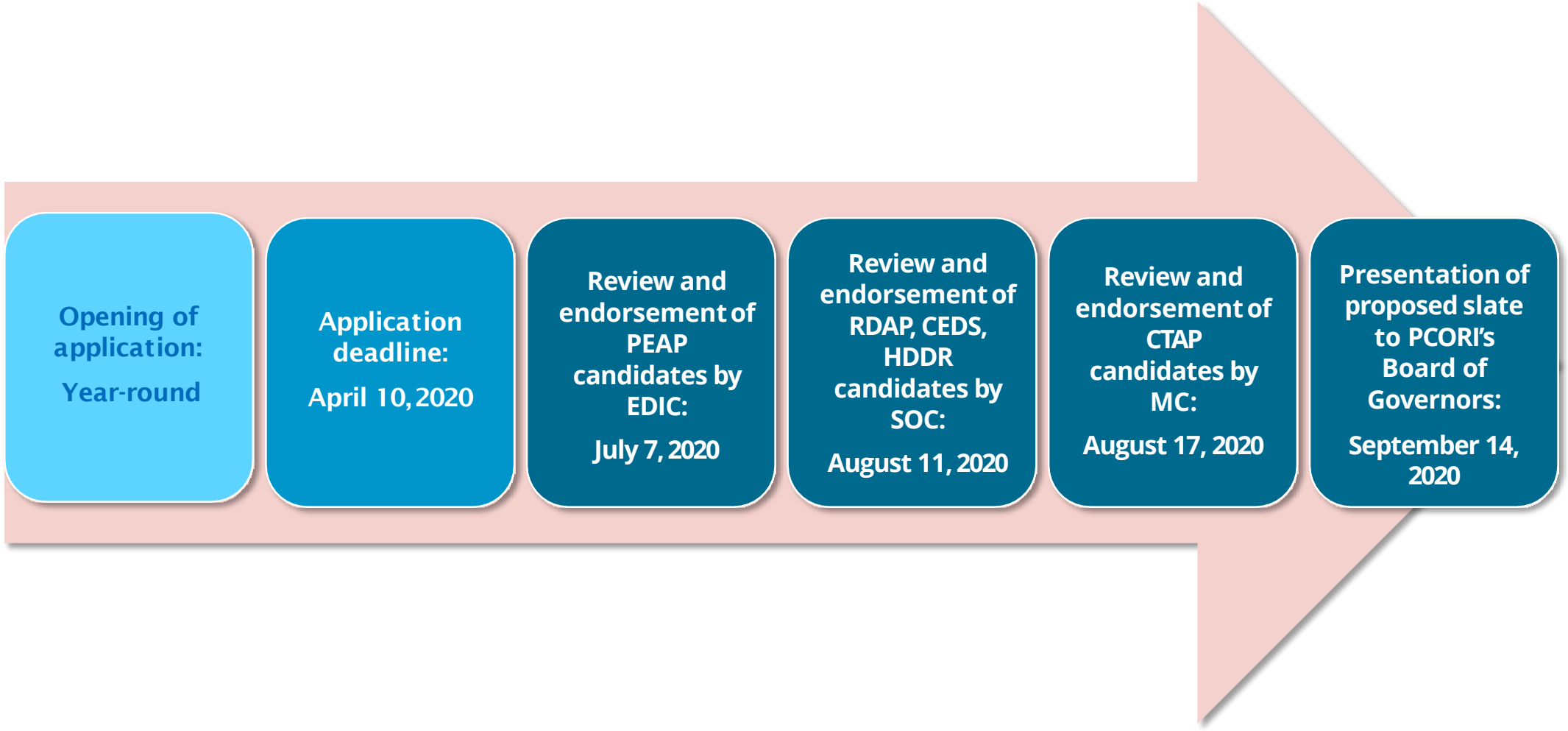
Approval of Advisory Panel Members, Chairs and Co-Chairs

Kristin Carman, MA, PhD
Director, Public and Patient Engagement

Steven Clauser, PhD, MPA
Program Director, Healthcare Delivery and Disparities Research

Stanley Ip, MD
Interim Program Director, Clinical Effectiveness and Decision Science

Advisory Panel Selection Timeline



Proposed Panelists

Advisory Panel	Applications Received	Number of New Panelists	Proposed New Chairs/Co-Chairs
Clinical Effectiveness and Decision Science	17	14	0
Clinical Trials	23	6	1
Healthcare Delivery and Disparities Research	28	4	2
Patient Engagement	65	4	2
Rare Disease	20	6	1
Total	153	34	6

Panelists Proposed by the SOC and MC

Steven Goodman, MD, MHS, PhD
Chair, Methodology Committee

Robert Zwolak, MD, PhD
Chair, Science Oversight Committee

Stanley Ip, MD
Interim Program Director, Clinical Effectiveness and Decision Science

Steven Clauser, PhD, MPA
Program Director, Healthcare Delivery and Disparities Research

Advisory Panel on Clinical Trials: Proposed Panelists

Name	Stakeholder Group	Primary Affiliation	Term Length*
Rowena Dolor	Researchers	Duke University Medical Center	3 years
Ilana Gareen	Researchers	Brown University School of Public Health	3 years
Larry Kessler	Researchers	University of Washington	3 years
William Tierney	Researchers	University of Texas at Austin, Dell Medical School	3 years
Michael L. Jones	Researchers	Brenau University, School of Nursing	3 years
Susan Lin	Patients, Caregivers, and Patient Advocates	Marymount University	3 years

*Or until replacements are appointed

Advisory Panel on Clinical Trials: Proposed Chair

Name	Stakeholder Group	Primary Affiliation	Term Length*
Catherine Crespi-Chun	Researchers	UCLA, Fielding School of Public Health	1 year

*Or until replacements are appointed

Advisory Panel on Rare Disease: Proposed Panelists

Name	Stakeholder Group	Primary Affiliation	Term Length*
Danielle Boyce	Patients, Caregivers, and Patient Advocates	National Organization for Rare Disorders	3 years
Laura Tosi	Clinicians	Children's National Hospital	3 years
Nancy Rose	Clinicians	American College of Medical Genetics and Genomics	3 years
Sarah Bacon	Patients, Caregivers, and Patient Advocates	Patient Advocate	3 years
Mathew Edick	Patients, Caregivers, and Patient Advocates	Michigan Public Health Institute	3 years
Salman Hussain	Industry	Charles River Associates	3 years

*Or until replacements are appointed

Advisory Panel on Rare Disease: Proposed Co-Chair

Name	Stakeholder Group	Primary Affiliation	Term Length*
Doug Lindsay	Patients, Caregivers, and Patient Advocates	Doug Says, LLC	2 years

*Or until replacements are appointed

Advisory Panel on Clinical Effectiveness and Decision Science: Proposed Panelists

Name	Stakeholder Group	Primary Affiliation	Term Length*
Adjoa Adofo Kyerematen	Policy Makers	CMMI	3 years
Mychal Weinert	Patients, Caregivers, and Patient Advocates	Med-EL Corporation	3 years
Lisa Goldman Rosas	Researchers	Palo Alto Medical Foundation Research Institute	3 years
Susan Johnson	Clinicians	Legacy Healthcare Services	3 years
William Bennett	Researchers	Indiana University, School of Medicine	3 years
Julie Eller	Patients, Caregivers, and Patient Advocates	Arthritis Foundation	3 years
Danielle Bargo	Industry	Flatiron Health	3 years

*Or until replacements are appointed

Advisory Panel on Clinical Effectiveness and Decision Science: Proposed Panelists, Cont.

Name	Stakeholder Group	Primary Affiliation	Term Length*
Samantha Harden	Researchers	Virginia Polytechnic Institute and State University	3 years
Karen Giuliano	Researchers	University of Massachusetts Amherst	3 years
Michael Philbin	Patients, Caregivers, and Patient Advocates	Nouryon	3 years
Kari Gali	Clinicians	Cleveland Clinic	3 years
Helen M. Beady	Patients, Caregivers, and Patient Advocates	American Heart Association	3 years
Joey Mattingly	Researchers	University of Maryland, Baltimore	3 years
Rick Rader	Researchers	Orange Grove Center	3 years

*Or until replacements are appointed

Advisory Panel on Healthcare Delivery and Disparities Research: Proposed Panelists

Name	Stakeholder Group	Primary Affiliation	Term Length*
Brandi Ring	Clinicians	Mile High OB/GYN	3 years
Kristina M. Cordasco	Researchers	VA Health Services Researcher, Center for the Study of Healthcare Innovation, Policy, and Practice	3 years
Varleisha Gibbs	Researchers	American Occupational Therapy Association, Inc.	3 years
Jeffrey Oliver	Patients, Caregivers, and Patient Advocates	Training by Seeds, LLC	3 years

*Or until replacements are appointed

Advisory Panel on Healthcare Delivery and Disparities Research: Proposed Co-Chairs

Name	Stakeholder Group	Primary Affiliation	Term Length*
Alicia Arbaje	Researchers	Johns Hopkins University	2 years
Jane Kogan	Payers	UPMC Insurance Services Division and Center for High-Value Health Care	2 years

*Or until replacements are appointed

Panelists Proposed by the EDIC

Lawrence Becker
Chair, Engagement, Dissemination, and Implementation Committee

Kristin Carman, PhD
Director, Public and Patient Engagement

Advisory Panel on Patient Engagement: Proposed Panelists

Name	Stakeholder Group	Primary Affiliation	Term Length*
Karen L. Fortuna	Researchers	Dartmouth College	3 years
Margarita Holguin	Patients, Caregivers, and Patient Advocates	Consulting Solutions Team, LLC	3 years
Alma McCormick	Patients, Caregivers, and Patient Advocates	Messengers for Health	3 years
Alan Richmond	Patients, Caregivers, and Patient Advocates	Community Campus Partnerships for Health	3 years

*Or until replacements are appointed

Advisory Panel on Patient Engagement: Proposed Chair and Co-Chair

Name	Stakeholder Group	Primary Affiliation	Term Length*
New Chair			
Gwen Darien	Patients, Caregivers, and Patient Advocates	National Patient Advocate Foundation	1 year
New Co-Chair			
Neely Williams	Patients, Caregivers, and Patient Advocates	Community Partners' Network	1 year

*Or until replacements are appointed

Board Vote

Call for a Motion to:	· **Approve** the proposed slates of members, positions and terms for the following Advisory Panels: CTAP, RDAP, CEDS AP, HDDR AP, and PEAP
Call for the Motion to be Seconded:	· **Second** the Motion · If further discussion, may propose an **Amendment** to the Motion or an **Alternative** Motion
Voice Vote:	· Vote to **Approve** the **Final** Motion · Ask for votes in favor, opposed, and abstentions

Proposed Amendments to Methodology Committee and Governance Committee Charters

Sharon Levine, MD

Chair, Governance Committee

Mary Hennessey, Esq.

General Counsel

Background

- PCORI's reauthorizing legislation shifted authority to appoint Methodology Committee members from the Comptroller General of the United States (GAO) to the PCORI Board of Governors
- The Governance Committee, with input from the Methodology Committee, deliberated on an appropriate governance framework for the Methodology Committee to enable the Board to exercise its appointment authority for Methodology Committee members
- The Governance Committee is recommending amendments to the Charters of the Methodology Committee and of the Governance Committee
  - The proposed amendments to the Methodology Committee Charter reflect the proposed governance framework for the Methodology Committee
  - The proposed amendments to the Governance Committee Charter align the language of the charter to the reauthorizing legislation

Proposed Amendments to MC Charter Reflecting Revised Governance Framework

- **Terms and structure:** 6-year terms with staggered-term structure
  - Provides for MC membership to be generally equally divided between new, experienced, and highly experienced (rotating off) MC members
- **Term limits**: MC members may be appointed to serve an additional 6-year term to the extent necessary to fulfill the functions of the MC, requirements of the authorizing law, and/or the needs of PCORI
  - No more than two full consecutive 6-year terms, if reappointed
- **Vacancies**: Appointment to the MC to fill a premature vacancy can be for the remainder of a predecessor's term
  - Preserves the staggered nature of the MC membership terms
- **Service until successor is appointed**: Confirms that an appointed MC member is eligible to serve until successor is appointed
  - Prevents gaps in valid MC membership

Proposed Amendments to Governance Committee Charter to Align with Reauthorizing Law

- The proposed amendments to the Governance Committee Charter delete language that is now outdated given changes in the reauthorizing legislation
  - The deleted language reflected that one of the responsibilities of the Governance Committee is to advise GAO regarding the process of appointment of MC members
  - This language is no longer accurate since PCORI's reauthorizing legislation transferred the appointment authority for MC members from GAO to PCORI's Board of Governors

Anticipated Next Steps

- If the Board approves the proposed amended MC Charter, plans for implementation will proceed, to include:
  - Planning for a regular cycle of appointments in odd years (e.g., 2021, 2023, etc.) to avoid confusion and competition with GAO's regular cycle of Board appointments which takes place in even years
  - Considering PCORI needs and principles, including scientific needs and conflict of interest principles (e.g., eligibility for PCORI funding)
  - Developing general framework for application and nomination, review of candidates, and Board appointment
  - Developing transition plan for current MC members

Board Vote

Call for a Motion to:	• **Approve the proposed amended Methodology Committee charter and the proposed amended Governance Committee charter**
Call for the Motion to be Seconded:	• **Second the Motion** • **If further discussion, may propose an Amendment to the Motion or an Alternative Motion**
Voice Vote:	• **Vote to Approve the Final Motion** • **Ask for votes in favor, opposed, and abstentions**

Limited Competition: Dissemination and Implementation PFA

Implementation of PCORI-Funded Patient-Centered Outcomes Research Results

Cycle 1 2020 Award Slate

Lawrence Becker

Chair, Engagement, Dissemination, and Implementation Committee

Joanna Siegel, SM, ScD

Director, Dissemination and Implementation Program

Cycle 1 2020 - Limited Competition Dissemination and Implementation PFA

Merit Review Criteria

Limited Competition PFA
1. Importance of research results in the context of the existing body of evidence
2. Readiness of the research results for implementation
3. Technical merit of the proposed implementation project
4. Project personnel and environment
5. Patient-centeredness
6. Patient and stakeholder engagement

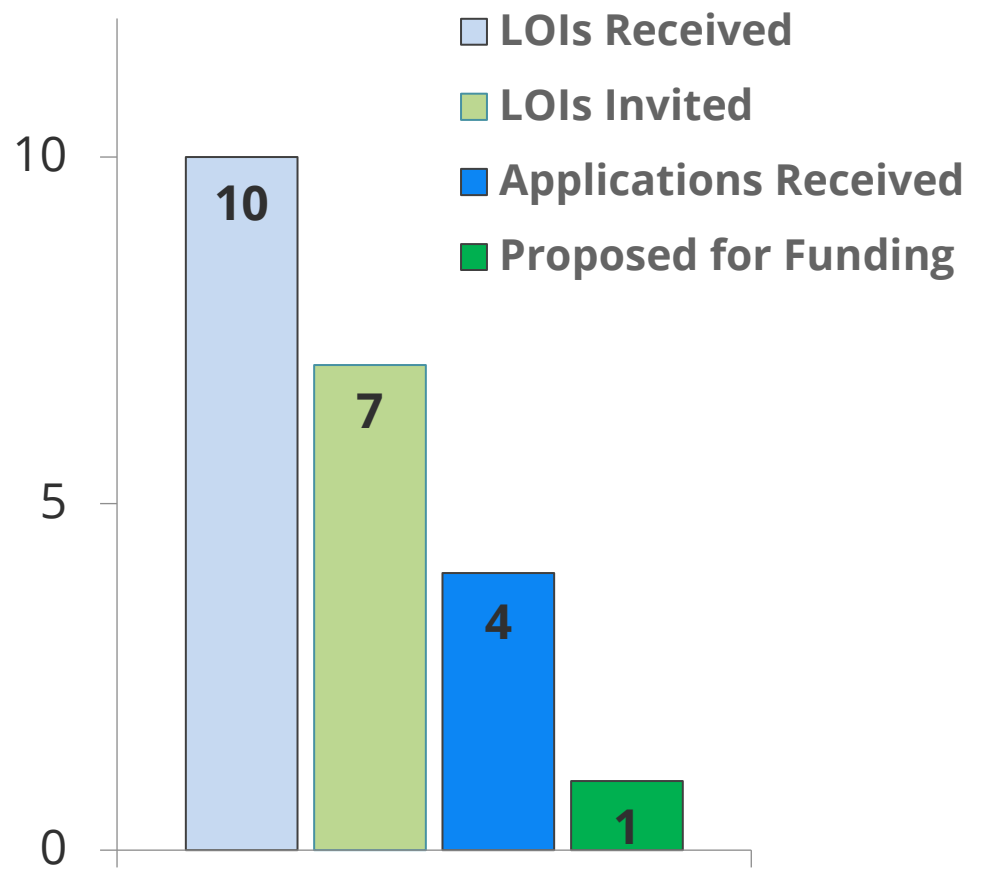
Cycle 1 2020 - Limited Competition Dissemination and Implementation PFA

Process Overview

- 10 Letters of Intent (LOIs) submitted
- 7 LOIs invited to submit a full application
- 4 applications received

Proposed funding slate is recommended by the Engagement, Dissemination, and Implementation Committee (EDIC)

- Proposing to fund 1 application (resubmission) out of the 4 received
- **Funding rate is 25 percent**



Cycle 1 2020 - Limited Competition Dissemination and Implementation PFA

Slate of 1 Recommended Project

Project Title
Implementation of Effective Home Oxygen Weaning Strategies in Premature Infants

Cycle 1 2020 - Limited Competition Dissemination and Implementation PFA

Slate of 1 Recommended Project

PFA	Amount Budgeted [per Year]	Proposed Total Award*
Implementation of PCORI-Funded Patient-Centered Outcomes Research Results	$9.0M	$1.3M

Note: Budgeted amount is for up to 3 cycles per year; this is the first cycle of 2020.

* All proposed projects, including requested budgets and project periods, are approved subject to a programmatic and budget review by PCORI staff and the negotiation of a formal award contract

Board Vote

Call for a Motion to:	• **Approve** funding for the recommended award slate from the Cycle 1 2020 Limited Competition Implementation of PCORI-Funded Patient-Centered Outcomes Research Results PFA
Call for the Motion to Be Seconded:	• **Second** the Motion • If further discussion, may propose an **Amendment** to the Motion or an **Alternative** Motion
Roll Call Vote:	• Vote to **Approve** the **Final** Motion • Ask for votes in favor, opposed, and abstentions

Executive Director's Report

Nakela L. Cook, MD, MPH
Executive Director

Celebrating Their Service
Outgoing Board Members 2010-2020

Lawrence Becker

Gail Hunt

Freda Lewis-Hall, MD

Grayson Norquist, MD, MSPH

Highlights from My First 150 Days

My First 150 Days

PCORI Operations	• Remote work and return to work plan • Establishing the Leadership Team • Diversity, Equity, and Inclusion Activities • Resuming Full Operations Post Re-authorization
Board of Governors and Committees	• Engaging with Committees • Strategic Planning
Stakeholder Engagement	• Listening Tour • Representing PCORI at diverse venues
Reauthorization Priorities	• Maternal Morbidity and Mortality • Intellectual and Developmental Disabilities • Cost Data Provision • Future of Methodology Committee
COVID-19 Response	• Funding • Tracking • Learning

My First 150 Days: PCORI Operations

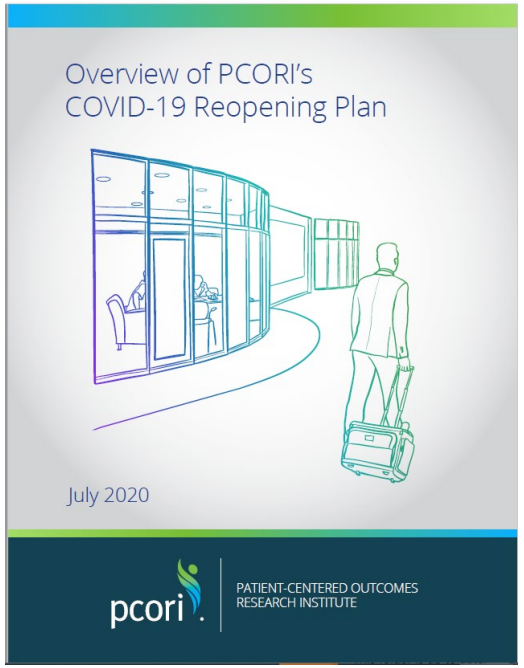
Remote Work & Return to Work Plans

Establishing the Leadership Team

Diversity, Equity, and Inclusion Activities

Resuming Full Operations Post-Reauthorization

Remote Work and Return to Work Plan



Staff functioning well remotely and with accelerated work in response to COVID-19

Guiding Principles

- Safety and Well-Being of PCORI's Workforce
- Flexible Approach for Needs and Circumstances of the Workforce
- Compliance with Requirements of Local and Federal Authorities
- Advanced Notice & Flexibility Between Phases

Gradual Staff Access

Phase 0	Phase 1	Phase 2	Phase 3	Phase 4
Workforce Members Teleworking	Workforce Members Teleworking; Only Designated Staff Permitted	Workforce Members Generally Teleworking; Initial Small Percentage Permitted	Telework Continues with Greater Office Accessibility	Workforce Members Expected to Return to Working in Office
	Flexible approach for timing with **at least** 4 weeks between			
Current State				Widely available effective prevention/ treatment; change in virus virulence or transmissibility

Establishing the Leadership Team

Josie Briggs, MD
Senior Advisor to the Executive Director

Steven Clauser, PhD, MPA
Program Director, Health Care Delivery and Disparities Research

Mary Hennessey, Esq.
General Counsel

Stanley Ip, MD
Interim Program Director, Clinical Effectiveness and Decision Science

Michele Orza, ScD
Chief of Staff

Tasha Parker
Director, Communications

Laura Lyman Rodriguez, PhD
Interim Chief Program Support Officer and Senior Advisor to the Executive Director

Jean Slutsky, PA, MSPH
Chief Engagement and Dissemination Officer

Regina Yan, MA
Chief Operating Officer

Diversity, Equity, and Inclusion Activities

Internal and External Activities

PCORI Virtual Annual Meeting
- Keynote Speaker – Dr. Lisa Cooper
- Panel – Standing up to Racism, Discrimination, and Bias: A Dialogue on Health and Healthcare Equity

Honest Conversation Dialogue
- Conversation for staff between the Executive Director and Dr. Josie Briggs

Staff Roundtables
- Roundtables with the Executive Director

Comprehensive Diversity, Equity, and Inclusion Initiative
- Formulating a Comprehensive Diversity, Equity, and Inclusion Initiative
- Internally and Externally Focused

Updating the Engagement Rubric
- Incorporating diversity and inclusion

Discussions in Other Venues

Venues include
- Academy Health Research Conference
- Stakeholder Roundtable
- PCORI Advisory Panels

PCORI Virtual Annual Meeting

Diversity, Equity, and Inclusion Activities

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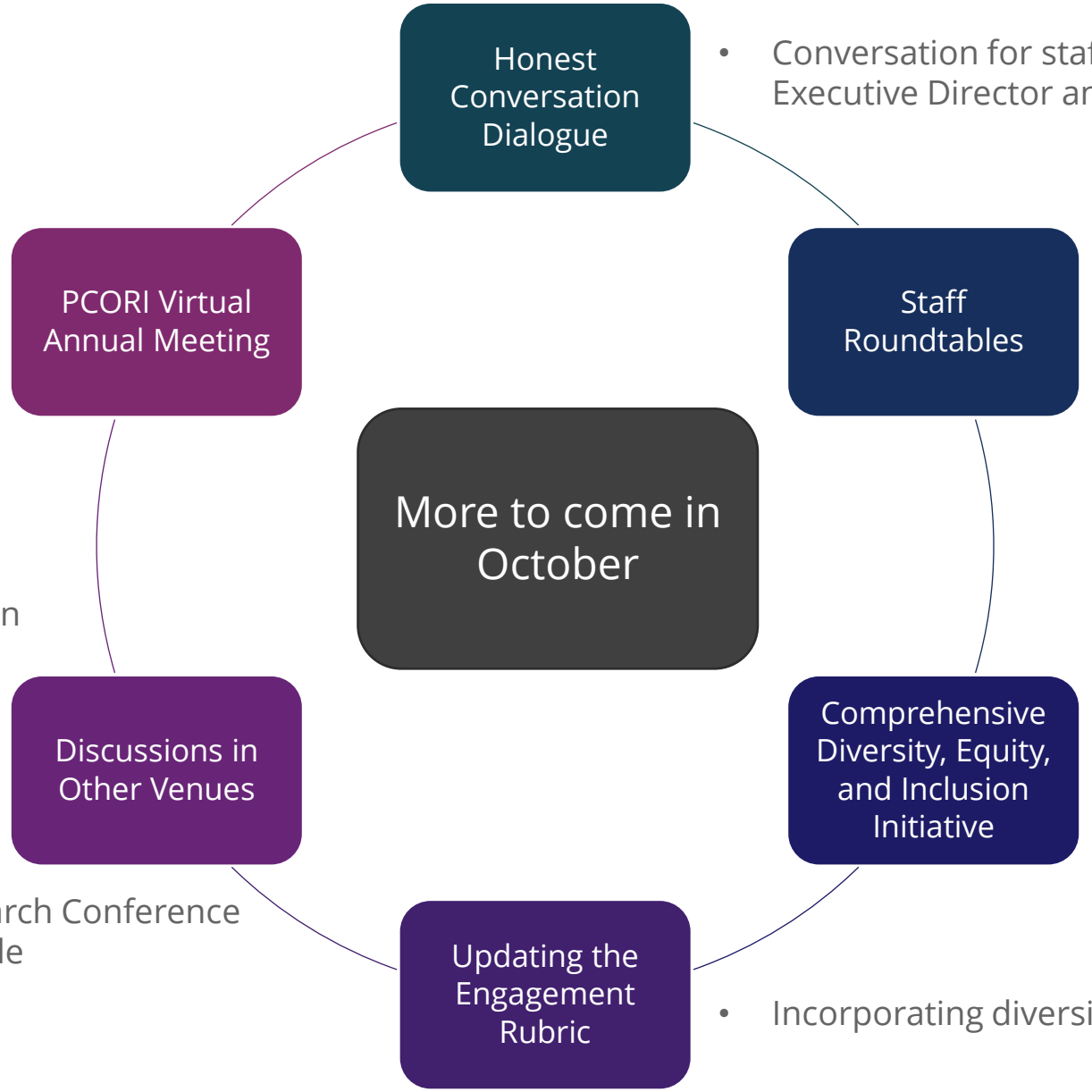


- Conversation for staff between the Executive Director and Dr. Josie Briggs
- Roundtables with the Executive Director
- Formulating a Comprehensive Diversity, Equity, and Inclusion Initiative
- Internally and Externally Focused
- Incorporating diversity and inclusion

My First 150 Days: Board of Governors & Committees

Engaging with Committees

- Engagement, Dissemination, and Implementation Committee
- Research Transformation Committee
- Science Oversight Committee
- Finance and Administration Committee
- Governance Committee
- Selection Committee
- Methodology Committee

Communications

- Building relationships
- Establishing regular communications

My First 150 Days: Listening Tour with Diverse Stakeholder Groups

Advisory Panels

- National Priorities
- Priorities in Legislation

Payers

Implementation of Cost Data Provision

Purchasers

Addressing Social Determinants of Health

Congressional Leaders

Data to Support Value in Health Care

- Eliminating Disparities
- Priorities in Legislation
- COVID-19 Response

Patients

- Patient-Driven Research
- Diversity & Inclusion
- Priorities in Legislation

My First 150 Days: Reauthorization Priorities

Implementing Provision on Cost/Economic Data

- Expanding PCORI's role in collecting and generating relevant evidence
- Focusing on a deliberate and transparent process for implementation

Developing New Priority Topics

- Maternal morbidity and mortality
- Intellectual and developmental disabilities

Maternal Morbidity and Mortality and Intellectual and Developmental Disabilities

Stakeholder Engagement

- Has begun
- Long-term
- Existing, new, diverse groups & lived experience

Funding Opportunity Development

Framing of Other Evidence Products (e.g., systematic reviews)

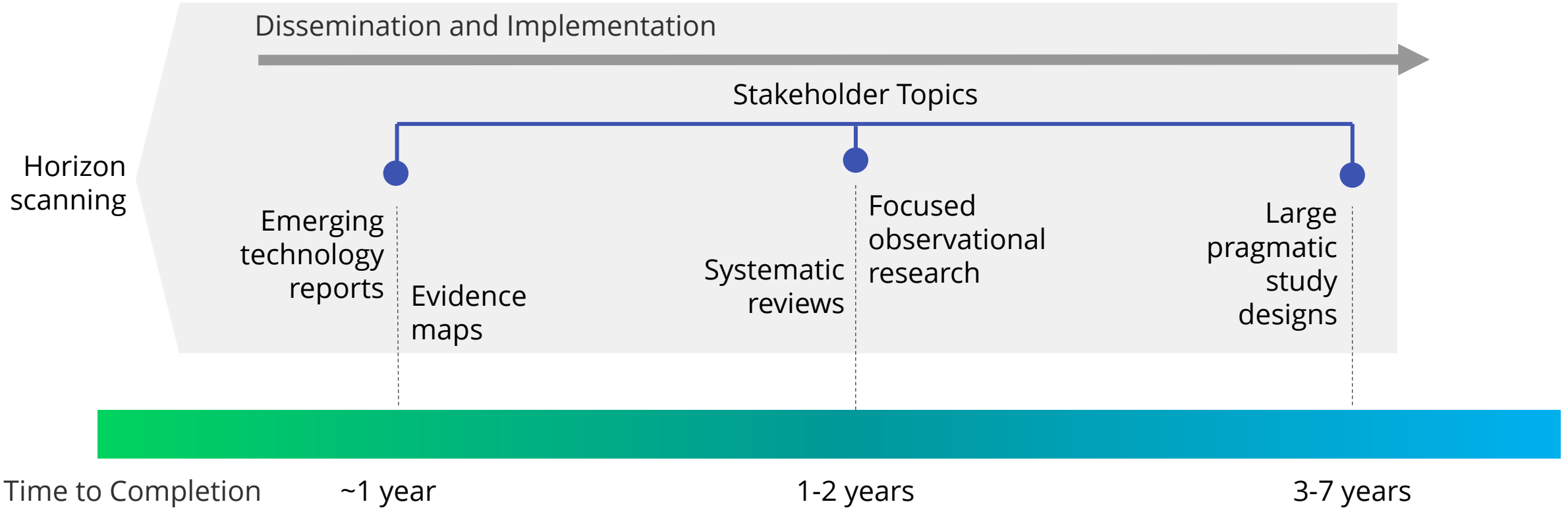
Implementation Plans for Data Collection

Special Areas of Emphasis:

- Increasing Access to and Continuity of Patient-Centered Maternal Care
- Improving Care for Individuals with Intellectual and/or Developmental Disabilities Growing into Adulthood

Balancing Long- and Short-term Activities

PCORI is providing a range of evidence products to meet patient and stakeholder needs, balancing long- and short-term research, and increasing D&I activities



Methodology Committee (MC)

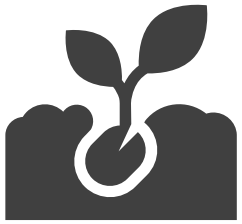
Charter Revisions Support PCORI

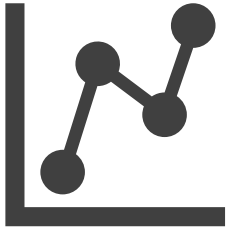
- Shifts authority to appoint MC members from GAO to PCORI Board of Governors
- 6-year terms with staggered-term structure
- Maximum of two 6-year terms to help meet needs of PCORI (and MC functions, authorizing law)
- Preserves staggered nature of the MC membership
- Prevents gaps in MC membership

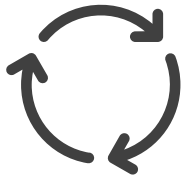
Methodology Committee Supports PCORI 2.0

- Diverse expertise to inform PCORI's activities
- Continue defining and updating methodological standards for patient-centered outcomes research
- Additional areas of focus to garner expertise of MC as it relates to activities of PCORI 2.0 (e.g., maternal morbidity and mortality, intellectual and developmental disabilities)

My First 150 Days: PCORI's COVID-19 Response







Funding

- HERO Research Program
- Targeted Funding
- Enhancements

Tracking

- Study Status
- Disruptions/Delays
- Adaptations
- Operational Pilots

Learning

- Operational Innovations
- Implications for Healthcare Delivery, Disparities, & Other Conditions Beyond Pandemic

COVID-19 Update

PCORI's Response to the COVID-19 Health Crisis

Many approaches to supporting critical work in these areas and more:

Awards

- Enhancements of existing awards
- Solicitation of new awards
- Healthcare worker registry/trial

Information Sharing

- COVID-19 Horizon Scanning
- PCORI Annual Meeting
- Webinars
- Collaboration with other funders

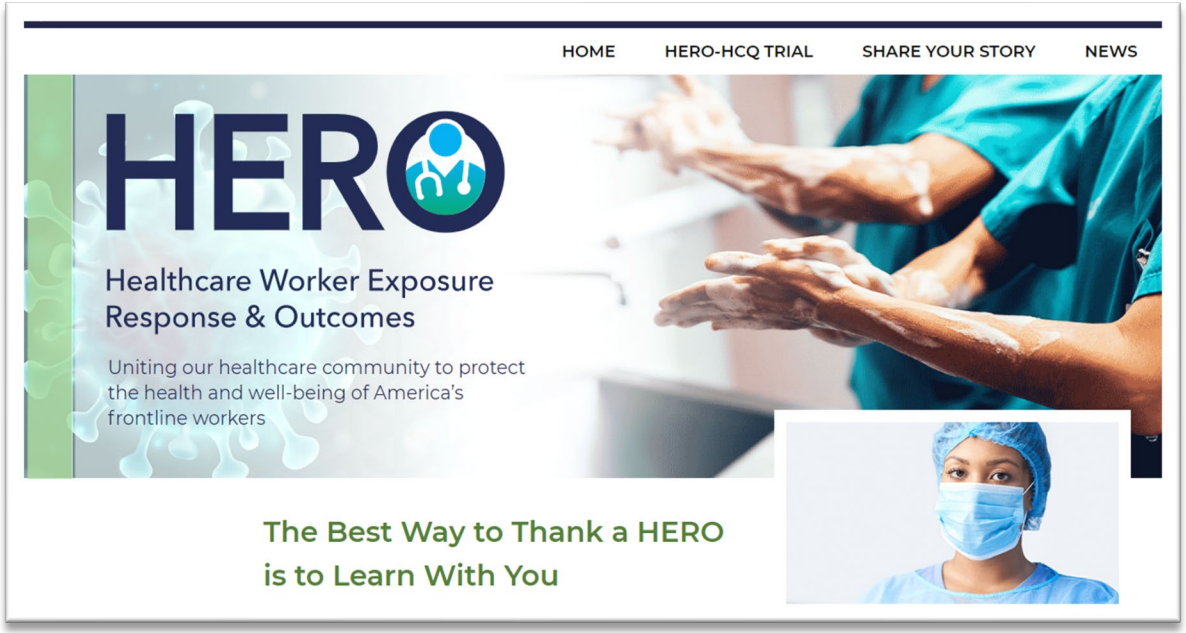
Adapting for Awardees and Applicants

- Adaptations to existing projects
- Extending application timelines

COVID-19 Funding Update

- The PCORI Board of Governors approved **up to $160M** in funding for COVID-19 related projects, enhancements, and adaptations.
- **Update**: as of 9/10/20, **$114M has been committed**
  - **$80M in Targeted awards** (10 projects)
    - HERO Registry and Trial
    - 9 awards awarded under the COVID-19 Targeted PFA
  - **$33M in Enhancements** (109 projects)
  - **$513K in Adaptations** (2 projects)
    - In addition, 14 Adaptations that did not require additional funding
    - Adaptations are expected to increase over the next several months

PCORI COVID-19 Funding to Date



www.heroesresearch.org

COVID-19 Targeted Funding

- Adaptations to healthcare delivery
- Impact on vulnerable populations
- Healthcare workforce well-being, management and training

COVID-19 Enhancement Projects

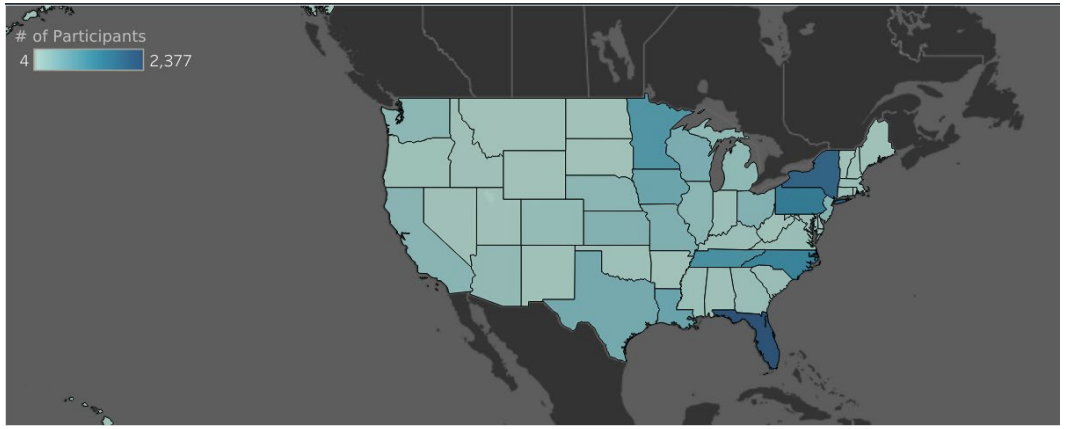
As of September 3rd, 2020

PCORI-Funded HERO Research Program

Principal Investigators: Adrian Hernandez, MD, MHS; Emily O'Brien, PhD (HERO registry); Susanna Naggie, MD (HERO-HCQ trial)
Awardee Institution: Duke University

HERO Registry

- Create community of healthcare workers (HCWs) at risk of COVID-19 infection
- Identify HCWs interested in engaging in clinical trials related to COVID-19
- Create dataset of basic clinical and environmental COVID-19 risk factors and clinical and emotional outcomes for analysis
- **16,984** HCWs enrolled



HERO-HCQ Trial

- Randomized clinical trial to evaluate efficacy of hydroxychloroquine (HCQ) to prevent COVID-19 clinical infection in at-risk HCWs
  - Targeted enrollment revised to 2,000 HCWs in response to changes in assessment of clinically meaningful effect size
  - Preserves 80% power to detect difference in prevention of infections
  - Remains one of the largest and most rigorous HCQ prophylaxis studies
- Secondary aims evaluate efficacy of HCQ to prevent viral shedding of SARS-CoV-2 among HCWs, and evaluate safety and tolerability of HCQ
- Ongoing scientific and media debate
- **1,312** HCWs enrolled from 35 enrolling sites

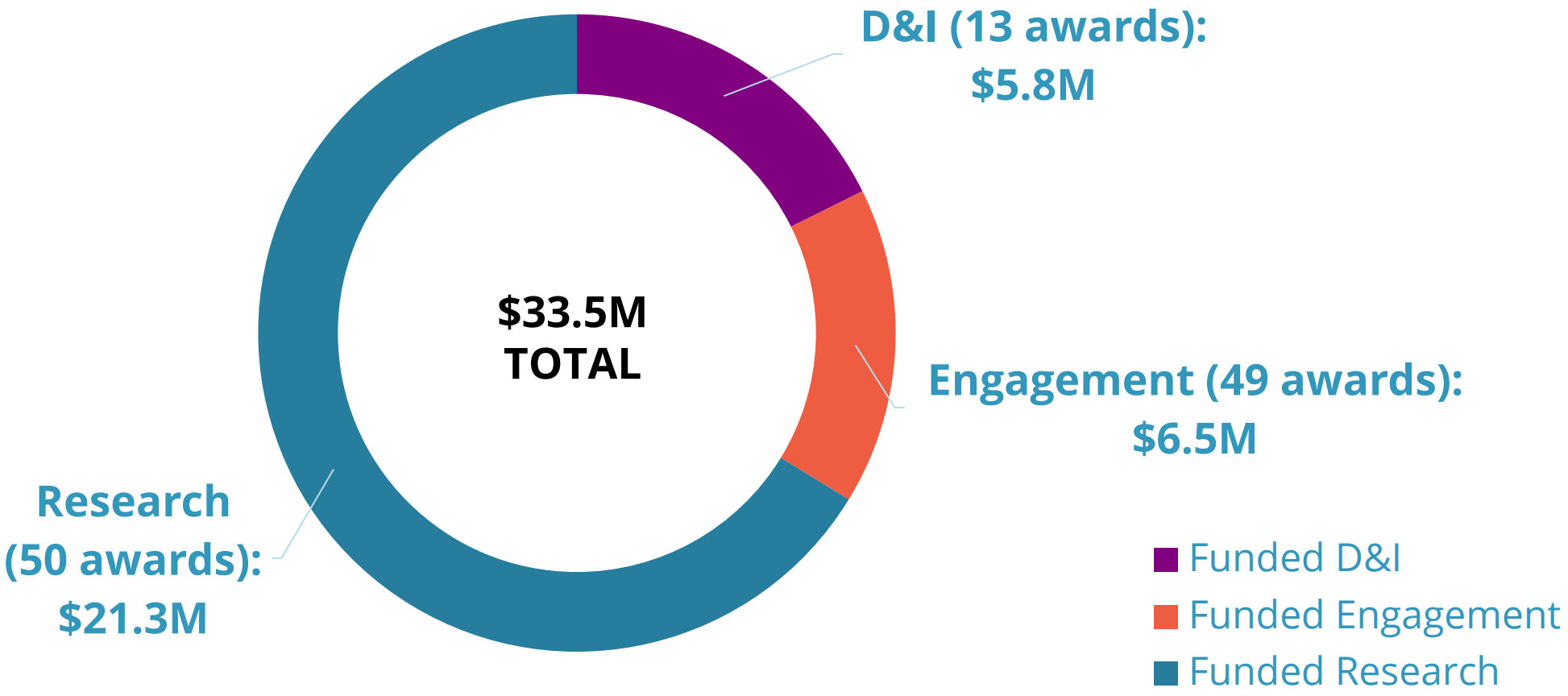
Oversight of the HERO-HCQ Trial

- **HERO-HCQ** trial oversight mechanisms include the following:
  - Trial conducted under Investigational New Drug Safety FDA requirements
  - Data Safety and Monitoring Board (DSMB) established by the sponsor
    - DSMB is engaged with other DSMBs of large COVID-19 related trials
    - Recommend continuation, revision or discontinuation of protocol
  - Trial subject to Institutional Review Board review
- Monitoring processes assure effectiveness, safety, or futility issues are addressed promptly
- Consistent with best practices for trial oversight, early termination is possible if indicated by either safety or futility
- PCORI Advisory Panel advises PCORI's Executive Director about overall funding and monitoring questions and issues
- PCORI contractual mechanism and processes reduce financial impact of early termination

COVID-19 Enhancements – Current PCORI Funding

Funded Enhancements Data

Last updated: September 3, 2020



COVID-19 Enhancements: Conditions and Interventions

Health Condition	Research Awards & Intervention Strategy										Dissemination & Imple-mentation Awards	Engagement Awards
	Drug	Other Clinical	Health Services	Tele-medicine	Training & Education	Screening	Tech-nology	Policy	Behavioral	N/A		
Cancer			■			■				■		■
Cardiovascular Diseases		■	■	■						■	■	■
Gastrointestinal Disorders			■									■
Genetic Disorders												■
Infectious Diseases							■				■	
Mental/Behavioral Health				■	■				■		■	■
Multiple/Comorbid Chronic Conditions			■									
Muscular & Skeletal Disorders										■		■
Neurological Disorders	■			■							■	
Nutritional & Metabolic Disorders	■		■	■	■			■			■	
Rare Disease											■	■
Reproductive & Perinatal Health			■	■						■	■	■
Respiratory Diseases							■				■	■
Other or Non-Disease Specific			■								■	■
Kidney Diseases										■		■
Blood Disorders												■
Trauma/Injuries											■	■

Comparing the Benefits and Harms of Medicines for Long-Term Treatment of Blood Clots – The ALTERNATIVE Study

Project Objectives

- Retrospective cohort study that compares benefits and harms of 5 treatment options for extended treatment of venous thromboembolism (VTE): warfarin and 4 new oral anticoagulants (NOACs): dabigatran, rivaroxaban, apixaban, and edoxaban
- Compares rates of recurrent VTE and bleeding:

  1) extended anticoagulation vs. no extended anticoagulation

  2) extended treatment with warfarin vs. NOACs

  3) rates of recurrent VTE and bleeding in patients on individual NOACs and in relevant subgroups

COVID-19 Enhancement

- COVID-19 appears to be associated with abnormal coagulation and may increase the risk of VTE and other thrombotic events. Understanding prevalence of post-hospitalization VTE and risk in key subgroups is of great interest to patients and clinicians.
- The research team will conduct an observational study that aims to answer questions about risk of COVID-19-related VTE broadly and in key patient subgroups, including older adults, those with a prior history of VTE, and those with severe COVID-19.

Principal Investigator: Margaret Fang, MD, MPH, University of California, San Francisco

Study Title: The Comparative Effectiveness of Warfarin and New Oral Anticoagulants for the Extended Treatment of Venous Thromboembolism

Research Project, Awarded 2016

Increasing Use of a Decision Aid about Immune-Blocking Medicines for People with Lupus

Project Objectives

- To implement findings from a completed PCORI-funded study that found using a decision aid helped patients with lupus choose a treatment option aligned with their values and preferences
- To integrate the decision aid as a part of patient care at 15 lupus clinics including training for doctors and staff on using the decision aid to facilitate shared decision making with the patient
- To update the decision aid to include additional information about lupus and immunosuppressive treatment options

COVID-19 Enhancement

- Patients with lupus may have a higher risk of serious complications from COVID-19. Additionally, many health systems have shifted from in-person care to telehealth.
- Adapts a decision aid that supports patients with lupus in making an informed decision about their treatment choice, for use in telehealth appointments.
- Adds two new modalities (smartphone app and website) and develops processes for delivering the decision aid for patients to complete in advance of their telehealth visit. The new telehealth-friendly modalities will reach 500 new patients with lupus at 15 sites nationwide.

Principal Investigator: Jasvinder Singh, MD, MPH, University of Alabama at Birmingham

Project Title: Implementation of DEcision-Aid for Lupus in Practice Settings for Shared Decision-Making (SDM): IDEAL

Implementation of Effective Shared Decision Making Approaches Project, Awarded 2018

Establishing a Patient-Led African Immigrant Health Research Consortium

Project Objectives

- Understand barriers, facilitators, and motivators to engaging African immigrant patients, caregivers, and groups in PCOR/CER
- Determine and prioritize their research interests and chronic disease concerns that can be addressed through PCOR/CER
- Develop three-year research agenda based on the prioritized chronic diseases, community assets, and research gaps and disseminate lessons learned, tools, and outcomes

COVID-19 Enhancement

- Document COVID-19-related health challenges facing African immigrants using Photovoice, a qualitative method in which participants express their points of view or represent their communities by photographing scenes that highlight research themes
- Produce knowledge to inform interventions engaging African immigrant patients in COVID-19 prevention, testing, and treatment
- Contribute knowledge needed to manage capacity at hospitals and healthcare systems to serve immigrants during a pandemic

Project Lead: Chioma Nnaji, MED, MPH

Multicultural AIDS Coalition

Project Title: Establishing a Patient-Led African Immigrant Health Research Consortium

Engagement Award project, Awarded 2019

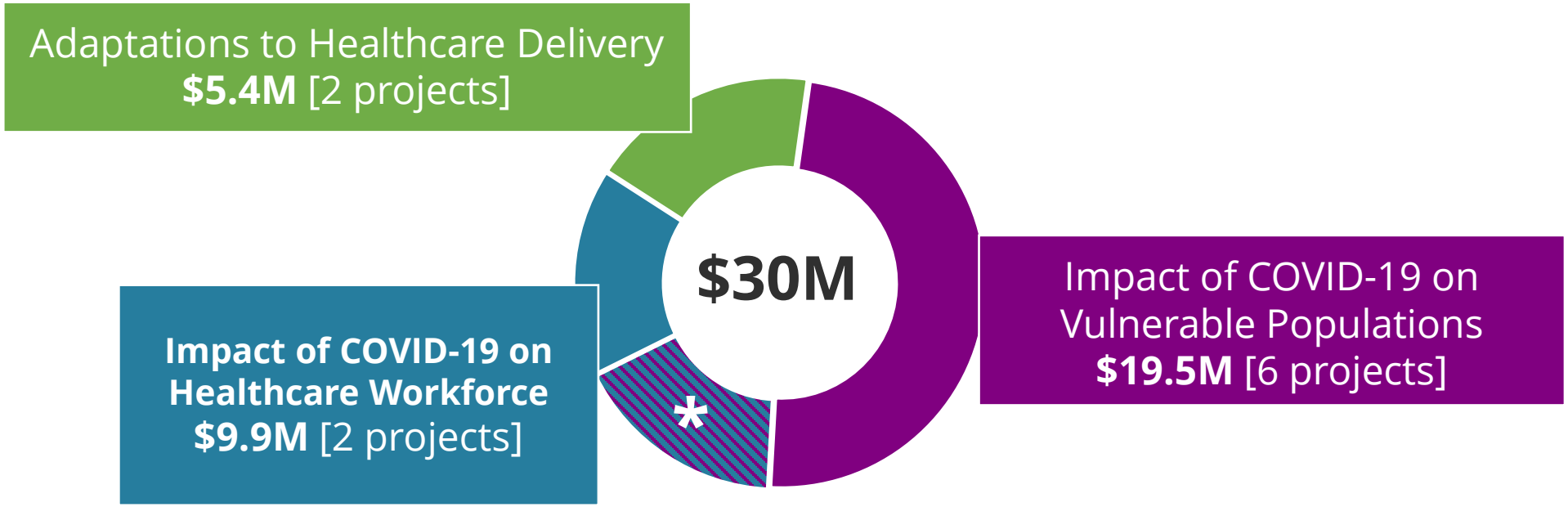
COVID-19 Targeted PFA

Slate of 9 Awards, approved in Aug/Sep 2020

Project Title
Comparative Effectiveness and Implementation of Best Practices to Prevent COVID Illness in Staff and People with Serious Mental Illness and Developmental Disabilities in **Congregate Living Settings**
Comparative Effectiveness of Single-Site and Scattered-Site Permanent Supportive Housing on Patient-Centered and COVID-19 Related Outcomes for People Experiencing **Homelessness**
Comparing Patient-Reported Impact of COVID-19 Shelter-in-Place Policies and Access to **Containment and Mitigation Strategies**, Overall and in **Vulnerable Populations**
Comparing Two Ways to Mitigate the Impact of the COVID-19 on **Mental Health** among Adults from **Underserved** and **Racial Minority Communities**
COVID-19 Project ECHO for **Nursing Homes**: A Patient-centered, Randomized-controlled Trial to Implement Infection Control and Quality of Life Best Practices
Evaluating the Comparative Effectiveness of **Telemedicine in Primary Care**: Learning from the COVID-19 Pandemic
Evaluating the Effectiveness and Implementation of an Automated **Remote Monitoring** Program for COVID-19 Patients
Impact of COVID-19-Related Medication Assisted Treatment Policy Changes on Patients with **Opioid Use Disorders**
Protecting the Mental and Physical Well-Being of **Frontline Healthcare Workers** During COVID-19

COVID-19 Targeted PFA
Slate of 9 Awards, approved in Aug/Sep 2020

- **$30M in <u>New</u> Targeted Awards** [9 projects]



*One study fit under two priority areas (Impact of COVID-19 on Vulnerable Populations, and on the Healthcare Workforce) and is reflected in both totals

Project Monitoring During COVID-19

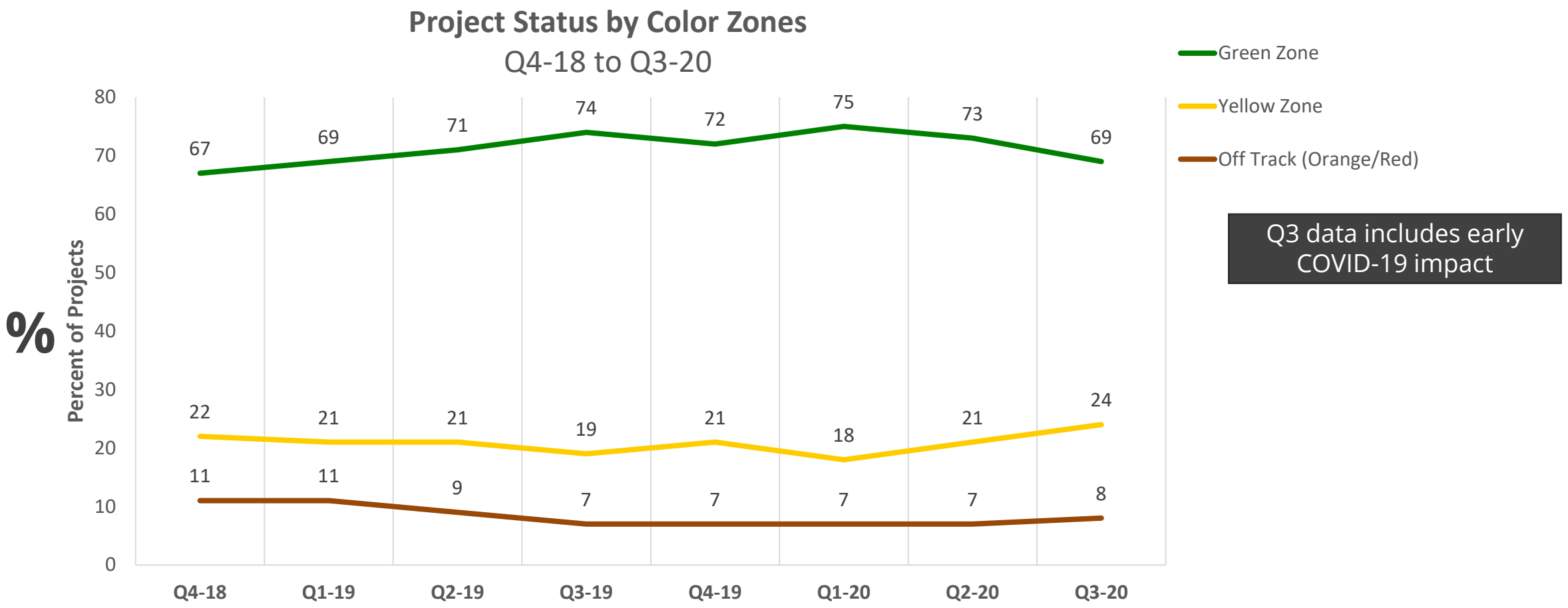
- We actively monitor our projects, support them to be successful, and classify research projects according to this scale. In addition to using this scale, we are also assessing disruptions and delays due to COVID-19.
  - In Q3-20, 241 studies were eligible for evaluation:
  - 166 are considered Green, and 75 projects are considered Yellow, Orange, or Red.

		Preview of Q3-20 Data:
Green	Highly likely to meet objectives as planned	166 (69%)
Yellow	May not be able to meet objectives within project period	57 (24%)
Orange	Will not meet objectives within the project period	17 (7%)
Red	Cannot meet its original objectives	1 (0.4%)

*Assessed on a quarterly basis, based on Milestones, Recruitment, and Program Officer assessment

Status of PCORI-Funded Research Projects:
% of projects in Yellow is increasing due to COVID-19 delays

We are monitoring trends and shifts in project status (most recent 8 quarters shown)



COVID-19 Disruptions/Delays to Research

- Among 75 projects that may not or will not meet objectives in the project period, 69 projects are experiencing COVID-related delays. Among those 69 projects:

Disruption/Delay Type	# (%)
Recruitment/activities significantly decreased	**39**/69 (57%)
The award is completely paused	**13**/69 (19%)
Intervention is being adapted	**13**/69 (19%)
No new recruitment; continuing follow-up	**11**/69 (16%)
Feasibility concern	**7**/69 (10%)
Follow-up is prohibited or significantly decreased	**5**/69 (7%)
Other non-enrollment related disruptions	**10**/69 (14%)

We will continue to collect this information and report it in the Dashboard materials

*Awards may have more than one type of delay/disruption

Looking Forward to My Next 150 Days

Future Funding Opportunities

- Next phase of COVID-19 response
- Health disparities
- Strategic vision for next phase for PCORnet
- Large phased clinical trial awards

Board of Governors

- Strategic Planning
- Welcoming new Board members

Engaging with Stakeholders

- National Priorities
- Research Agenda
- Maternal Mortality
- Intellectual and Developmental Disabilities
- Cost Data Outcomes

Operational Innovations

- Evaluating and enhancing operations
- Learning from operational pilots
  - e.g., Virtual Merit Review, Expedited Solicitation Cycles
- Operating models for future

BREAK

We will return at 3:30 PM ET

Join the conversation on Twitter via @PCORI

Welcome Back

Christine Goertz, DC, PhD

Chairperson, Board of Governors

Approving PCORI's Draft "Principles for the Consideration of the Full Range of Outcomes Data" for Posting for Public Comment

Andrew Hu

Director, Engagement, Public Policy

Joanna Siegel, SM, ScD

Director, D&I Program

Outline for Today's Presentation

Setting the Context

- What is in the reauthorizing law
- Congressional and stakeholder intent

Implementation Plan

- Phases and Timing
- Opportunities for Public Comment

Consideration of Approval

- Preliminary Input
- Proposed "Principles"
- Board discussion and consideration of approval for posting for public comment

Overview of Reauthorizing Language

- 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 health care services, but also patient centric-measures of cost and burden. Potential burdens and economic impacts include:
    - Medical out-of-pocket costs, including health plan benefit and formulary design, non-medical costs to patients and families, including caregiving, effects on future costs of care, workplace productivity and absenteeism, and healthcare utilization.
- PCORI is **still prohibited** from developing quality adjusted life year (QALY) measures and conducting cost-effectiveness analysis via our authorizing legislation.
- To address the priorities and concerns of payers, purchasers, patient and consumer organizations, and the life sciences industry, Congress worked to balance the need to capture both traditional and patient-centric measures.

Congressional and Stakeholder Intent

Congress and stakeholders had very strongly-held positions related to PCORI's role in conducting cost-effectiveness analyses or considering cost/value as an outcome of our research. This remains a sensitive subject among stakeholders and of strong interest to members of Congress.

- The language is a compromise between pro cost-effectiveness analysis advocates and those who believe PCORI should remain focused on clinical and patient-centered aspects of decision-making

- This compromise language **does not** remove statutory prohibitions on PCORI establishing cost-per quality adjusted life year (QALY) thresholds or allow PCORI to conduct cost-effectiveness analyses

Early Stakeholder Input

Summary of the key take-aways for implementation include:

- Ensure **transparency**, notably patient engagement, throughout implementation
- Consider the **full range of treatment options**
- Need to **develop standards** around identifying and capturing patient-centric cost data
- **Concerns around the use/misuse** of cost data that could lead to cost-effectiveness or inappropriate value assessment
- Hope to **expand beyond traditional health economic perspectives** on cost/value

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

Timing for Implementation

Pillar 1 – Developing Guidance for Applicants

- Draft "Principles" to be considered by Board of Governors for posting for public comment in Sept 14-15 meeting
- 60-day Public Comment Period (Close 11/13)
- "Townhall" Webinars Scheduled for October 5th and 6th
- Input received on "Principles" will inform development of guidance for applicants
- Present revised "Principles" to be considered for approval by Board of Governors during Feb/Mar 2021

Pillar 2 – Methodology Standards

- Initiate process to update Methodology Standards in December 2020 or January 2021 (Following public input)

Pillar 3 – Uses of Data

- Hold initial convenings in November or December 2020
- Ongoing activities

PCORI intends to follow a very deliberate and transparent process throughout each phase of implementation with opportunities for public input at each phase.

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 for the updating of PCORI's Methodology Standards
- These principles should not be viewed as standards and methods

Early Feedback on Proposed Principles

Staff sought input from PCORI's Advisory Panels, Methodology Committee, as well as the EDIC, RTC and SOC throughout the summer

In **June**, staff presented background on this new mandate to the PEAP, CEDS, HDDR and RDAP

July 7: Staff first presented the proposed principles to the EDIC for input

July 16: Staff first presented the proposed principles to the RTC for input

August 4: Staff had a follow-up discussion with the EDIC for further reflection and discussion

August 11: Staff first presented the proposed principles to the SOC for input

Types of Input Received on Proposed "Principles"

- Need to clarify the definitions of "relevant outcomes" to patients and stakeholders
  - Note that patient cost will vary based on many factors, such as insurance coverage
  - Need to clarify that many payers and providers, as well as patients, face important decisions which should be informed by consideration of a full range of outcomes
  - Ensure data that are collected in the course of a study are made public

- Need to further clarify the distinctions between the "capture of data", "cost analyses", and the "conduct of cost-effectiveness"
  - Need to define scope of cost burden and economic impact data that is permissible to collect
  - Establish whether all PCORI-funded studies need to capture this type of data
  - Provide examples of the types of analyses PCORI would permit under this mandate and define the limits

Open Questions for Input

1. What are the advantages/disadvantages of a requirement for all PCORI-funded research to capture cost burden and economic impact data? What are reliable indicators of scenarios/ types of studies where the capture of this data will not contribute to overall importance or value of the research study findings?

2. Given the limits set in PCORI's authorizing statute, it is clear that **data collection** is in scope, and that **cost-effectiveness analysis** is out of scope. Understanding that there are circumstances where additional analysis of data may benefit – but that economic analysis should not be a primary goal of PCORI-funded studies – we seek input on the type of analyses that will benefit stakeholders, and the specific uses and advantages of each type of analysis.

Principles Proposed as a Basis for Developing Guidance for Applicants

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

Principle #1: Outcomes Important to Patients and Caregivers

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

- Principle reiterates the need for patient and caregiver engagement when identifying outcomes
- Identifies Economic/Cost components potentially important to patients:

- **Patient burden:**
  - Time in hospital
  - Time home from work and usual activities
  - Cost/time for transport
  - Childcare costs when seeking care
  - Out-of-pocket costs (copays and deductible; items not covered such as drugs or care providers)
- **Caregiver burden:**
  - Hours spent caregiving
  - Foregone wages
- **Utilization:** Without associated costs.
- **All substituted utilization/cost**

Principle #2: Outcomes Relevant to Other 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 long-term impact on patients.

- Principle differentiates the outcomes relevant to stakeholders from those important to patients and caregivers
- Provides example of why cost/economic impact data may be useful (i.e., non-inferiority study)
- Identifies Economic/Cost components potentially relevant to other stakeholders:

- **Cost of treatment/intervention**
  - Staff time
  - Costs of medication
  - Changes in medication dosage
- **Utilization:** Without associated costs.
- **Costs associated with changes in utilization**
  - Costs of increased, decreased or shifted visits of different types (ED, primary care)
  - Costs of changes in length of stay
  - Shifts in care site (e.g., outpatient versus inpatient)
- **Costs of establishing/implementing new intervention (program costs)**
- **Employer burden:**
  - Absenteeism
  - Reduced productivity from presenteeism

Principle #3: Guidance on 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.

- Principle states that PCORI will not fund studies where cost and economic impacts are the primary outcome
- Directs applicants to justify why they choose to – or not to – collect relevant cost and economic data
- Applicants should consider the feasibility of capturing these data when submitting research proposals
- The collection of data must be relevant to the specific research study

Principle #4: Guidance on Allowable 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.

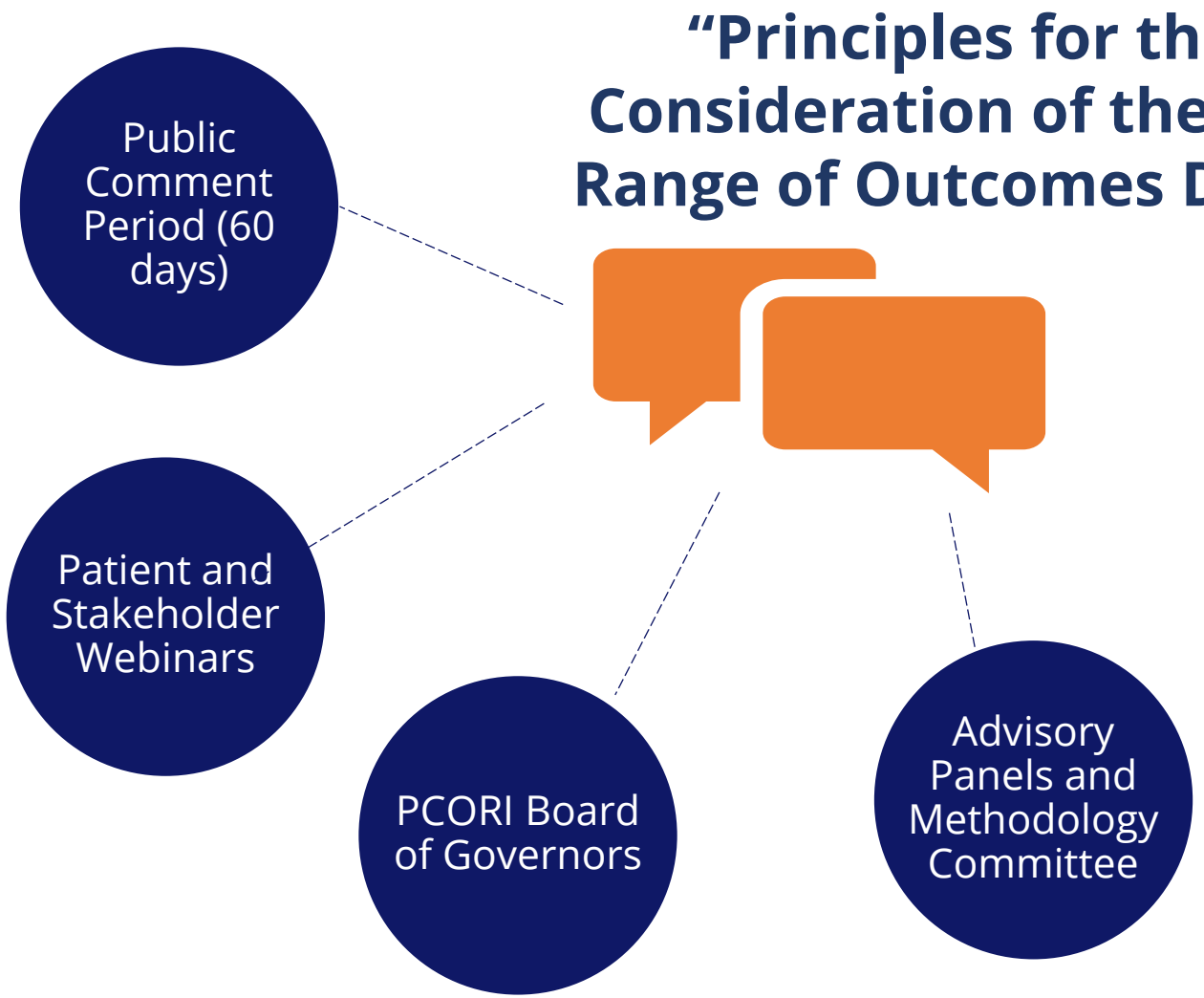
- Principle allows for the measurement of cost elements and/or the conduct of limited analysis, as appropriate
- Principle clearly defines that cost-effectiveness analyses **will not** be supported
  - Analyses **may not** aggregate findings on health outcomes with findings on economic impacts – as in a cost-effectiveness ratio
  - Studies **should not** be designed in such a way as to inform resource allocation decisions by aggregating and summarizing comparisons between alternative interventions.

Open Questions for Input

1. What are the advantages/disadvantages of a requirement for all PCORI-funded research to capture cost burden and economic impact data? What are reliable indicators of scenarios/ types of studies where the capture of this data will not contribute to overall importance or value of the research study findings?

2. Given the limits set in PCORI's authorizing statute, it is clear that **data collection** is in scope, and that **cost-effectiveness analysis** is out of scope. Understanding that there are circumstances where additional analysis of data may benefit – but that economic analysis should not be a primary goal of PCORI-funded studies – we seek input on the type of analyses that will benefit stakeholders, and the specific uses and advantages of each type of analysis.

Next Steps: Opportunities for Input on Principles



Consideration by the Board for Approval for Posting
September 14th

Possible Start of Public Comment Period
September 14th

Patient & Stakeholder Webinars
October 5th and 6th

Possible Close of Public Comment Period
November 13th

Presentation of Revised Principles to the Board for Consideration of Approval
Feb. or Mar. of 2021

Patient and Stakeholder Webinar Series

This webinar series will be held on October 5th and 6th and will allow for the public to provide input on the types of economic and cost data that are relevant to patients, consumers and stakeholders.

Panel 1 (Monday, October 5th from 2:30 – 4 PM)	Panel 2 (Tuesday, October 6th from 2:30 – 4 PM)
• Patient Advocate • Condition Specific Organization • Caregiver • Consumer Group • Disability Rights Advocate	• Payer • Purchaser • Life Sciences Industry • Provider • Health System

Discussion

Board Vote

Call for a Motion to:	• **Approve** the Draft Principles for the Consideration of the Full Range of Outcomes Data for Posting for Public Comment
Call for the Motion to be Seconded:	• **Second** the Motion • If further discussion, may propose an **Amendment** to the Motion or an **Alternative** Motion
Roll Call Vote:	• Vote to **Approve** the **Final** Motion • Ask for votes in favor, opposed, and abstentions

Closing Remarks

Wrap Up and Adjournment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