

Board of Governors Meeting

via Teleconference/Webinar

March 16, 2021

1:00 PM – 3:30 PM

Welcome and Call to Order

Christine Goertz, DC, PhD

Chairperson, Board of Governors

Agenda

- | | |
|------------------|--|
| 1:00-1:05 | Welcome, Call to Order and Consider for Approval: February 9, 2021 Board Minutes |
| 1:05-2:00 | Consider for Approval: Research and Dissemination & Implementation Award Slates |
| 2:00-3:00 | Consider for Approval: Research Topics for Targeted PFAs for Cycle 2 2021 |
| 3:00-3:30 | Consider for Approval: Principles for the Consideration of the Full Range of Outcomes Data in PCORI-Funded Research |
| 3:30 | Wrap-up and Adjournment |

Board Vote

Call for a Motion to:

- **Approve** the Minutes of the February 9, 2021 Board of Governors meeting

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

Overview of Award Slates

Nakela L. Cook, MD, MPH

Executive Director

Overview of Award Slates to be Considered for Approval Today

The following five award slates will be considered for approval:

PFA Type	Amount	PFA
Research	\$18.4M	Cycle 2 2020 Targeted PFA: Observational Analyses of Second-Line Pharmacological Agents in Type 2 Diabetes
Research	\$19.0M	Cycle 2 2020 Targeted PFA: Limited Competition PCORI Funding Announcement: Conducting Rare Disease Research using PCORnet®
Research	\$5.3M	Additions to the Cycle 1 2020 Broad Award Slate
D&I	\$3.7M	Cycle 2 2020 Limited Competition D&I PFA
D&I	\$2.1M	Cycle 2 2020 Implementation of Effective Shared Decision Making Approaches in Practice Settings PFA
Total	\$48.5M	

FY2021 Funding Cycles in Context

Two Targeted PFA Award Slates



LOIs, Applications, and Funding Rates were generally higher than historical averages:

Targeted PFAs	Historical Average*	Cycle 2 2020 Diabetes Observational		Cycle 2 2020 Rare Disease Using PCORnet		Cycle 3 2020 Suicide Prevention	
LOIs Submitted	15	24		22		29	
LOIs Invited (% of LOIs Submitted)	73%	22	92%	14	64%	16	55%
Applications Submitted (% of LOIs Invited)	73%	18	82%	13	93%	13	81%
Applications Proposed for Funding (% of Applications Submitted)	21%	4	22%	4	31%	TBD	

* Historical Average based on experience from 2018-2020

FY2021 Funding Cycles in Context

Deferrals from Cycle 1 2020 Broad PFAs



LOIs, Applications, and Funding Rate were similar to historical averages:

Broad PFAs	Historical Average*	Cycle 1 2020 Broad PFAs		Cycle 1 2020 Deferred Broad PFAs		Cycle 3 2020 Broad PFAs	
LOIs Submitted	184	170		N/A		202	
LOIs Invited (% of LOIs Submitted)	51%	88	52%	N/A		112	55%
Applications Submitted (% of LOIs invited)	75%	47	53%	19	22%	84	75%
Applications Proposed for Funding (% of Applications Submitted)	15%	8	17%	2	11%	TBD	

* Historical Average based on experience from 2018-2020

Note: Cycle 1 2020 Broad PFAs including Deferred applications had a 15% funding rate, same as the historical average

Today's Proposed Research Commitments in Context of FY2021 Commitment Plan



Research PFAs Funded in This Cycle	Commitment Target for Research in FY2021	Cumulative FY2021 Research Funding, with today's slates	Remaining Available for Research in FY2021
• Cycle 1 2020 Deferred Broad PFAs • Cycle 2 2020 Targeted PFAs: • Diabetes Observational • Rare Disease Using PCORnet	\$290M	\$84M	\$206M

- The following PFAs remain to be funded in FY2021:
 - Cycle 3 2020 Broad PFAs (with two Special Area of Emphasis topics)
 - Cycle 3 2020 Phased Large Awards for Comparative Effectiveness Research (PLACER)
 - Cycle 3 2020 Suicide Prevention: Brief Interventions for Youth
 - COVID Targeted PFA

FY2021 Funding Cycles in Context

Cycle 2 2020 Limited Competition D&I PFA



LOIs, Applications, and Funding Rate were similar to historical averages:

Limited Competition (LC) D&I PFAs to be Funded in FY2021	Historical Average	Cycle 2 2020 D&I LC PFA		Cycle 3 2020 D&I LC PFA		Cycle 1 2021 D&I LC PFA	
LOIs Submitted	13	11		6		6	
LOIs Invited (% of LOIs Submitted)	77%	8	73%	4	67%	4	67%
Applications Submitted (% of LOIs invited)	70%	8	100%	3	75%		
Applications Proposed for Funding (% of Applications Submitted)	29%	4*	50%	TBD			

*Includes one small award approved under authority of the Chief Engagement and Dissemination Officer

Note: Historical Average based on experience from 2018-2020

FY2021 Funding Cycle in Context

Cycle 2 2020 Implementation of Effective Shared Decision Making Approaches in Practice Settings PFA



LOIs, Applications, and Funding Rate were similar to historical averages:

Shared Decision Making (SDM) D&I PFAs to be Funded in FY2021	Historical Average*	Cycle 2 2020 D&I SDM PFA	
LOIs Submitted	7	2	
LOIs Invited (% of LOIs Submitted)	57%	1	50%
Applications Submitted (% of LOIs invited)	83%	1	100%
Applications Proposed for Funding (% of Applications Submitted)	60%	1	100%

* Historical Average based on experience from 2018-2020

Today's Proposed D&I Commitments in Context of FY2021 Commitment Plan



Dissemination & Implementation PFAs Funded in This Cycle	Commitment Target for D&I Awards in FY2021	Cumulative FY2021 D&I Award Funding with today's slates	Remaining Available for D&I Awards in FY2021
Cycle 2 2020: - Limited Competition - Shared Decision Making	\$28M	\$6M	\$22M

D&I PFAs Include:

- 1) Limited Competition PFA: Implementation of PCORI-Funded Patient-Centered Outcomes Research Results
- 2) Shared Decision Making Approaches in Practice Settings
- 3) Implementation of Findings from PCORI's Major Research Investments

- The following PFAs remain to be funded in FY2021:
 - Cycle 3 2020 Limited Competition D&I PFA
 - Cycle 1 2021 Limited Competition D&I PFA

Research Award Slates

Barbara McNeil, MD, PhD

Chair, Selection Committee



Observational Analyses of Second-Line Pharmacological Agents in Type 2 Diabetes

Cycle 2 2020 Award Slate

Kim Bailey, MS

Interim Associate Director,
Clinical Effectiveness and Decision Science

Cycle 2 2020 – Observational Analyses of Second-Line Pharmacological Agents in Type 2 Diabetes PFA

PFA Overview



- **Objective of this PFA:**
 - Fund high-quality studies using observational designs that compare the effectiveness of newer (SGLT2 inhibitors and GLP-1 receptor agonists) versus older (DPP-4 inhibitors and sulfonylureas) second-line pharmacological agents used to treat type 2 diabetes among individuals at moderate cardiovascular risk
- **Posted Funds:** Up to \$20 million
- **Total Direct Costs per Project:** Up to \$4 million
- **Maximum project period:** 36 months

Cycle 2 2020 –Observational Analyses of Second-Line Pharmacological Agents in Type 2 Diabetes PFA

Slate of 4 Recommended Projects



Project Title	
Second-line Therapies for Patients with Type 2 Diabetes (T2D) and Moderate Cardiovascular Disease Risk	
Comparison of Type 2 Diabetes Pharmacotherapy Regimens Using Targeted Learning	
BESTMED: oBservational Evaluation of Second line Therapy Medications in Diabetes	
The Comparative Effectiveness and Safety of Four Second-Line Pharmacological Strategies in Type 2 Diabetes Study (The CER-4-T2D study)	
Total Funds Requested:	\$18.4M

Resubmissions in Bold

Note: 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 slate of awards from the Cycle 2 2020 Observational Analyses of Second-Line Pharmacological Agents in Type 2 Diabetes 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

Voice Vote:

- Vote to **Approve** the **Final** Motion
 - Ask for votes in favor, opposed, and abstentions

Conducting Rare Disease Research Using PCORnet®

Cycle 2 2020 Award Slate

Penny Mohr, MA

Senior Advisor, Emerging Technology
and Delivery System Innovation Research Initiatives

Cycle 2 2020 – Conducting Rare Disease Research Using PCORnet®

PFA Overview



- **Objectives of this PFA:** PCORI seeks to fund new research that meets the following two primary objectives:
 - Utilize PCORnet resources to perform an observational research study that will answer one or more important research questions about the care of patients with rare diseases
 - Enhance the capabilities for the conduct of multi-site rare disease research by creating partnerships, methods, tools, and linkages that will facilitate future comparative effectiveness studies
- **Posted Funds:** \$25 million
- **Total Direct Costs per Project:** \$3.5 million
- **Maximum project period:** 3 years

Cycle 2 2020 – Conducting Rare Disease Research Using PCORnet®

Slate of 4 Recommended Projects



Project Title	
Preserving Kidney Function in Children with Chronic Kidney Disease (PRESERVE)	
Comparative Effectiveness Research for Neuroendocrine Tumors (CER-NET)	
Comparative effectiveness of palliative surgery versus additional anti-seizure medications for Lennox-Gastaut Syndrome	
Utilizing PCORnet to support transition from pediatric to adult centered care and reduce gaps in recommended care in patients with congenital heart disease	
Total Funds Requested:	\$19M

Resubmissions in Bold

Note: 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 slate of awards from the Cycle 2 2020 Conducting Rare Disease Research Using PCORnet® 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

Voice Vote:

- Vote to **Approve** the **Final** Motion
- Ask for votes in favor, opposed, and abstentions

Additions to the Cycle 1 2020

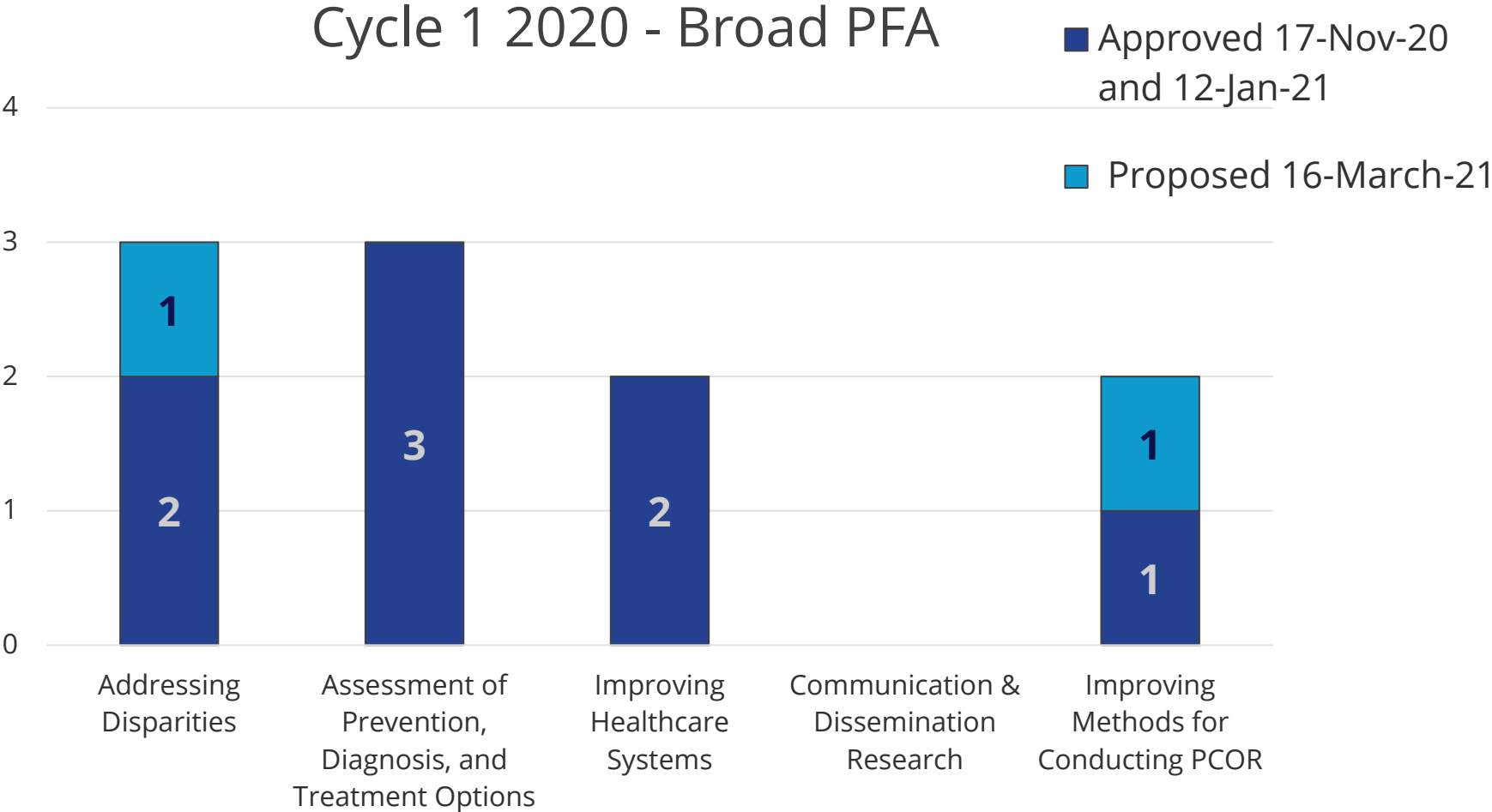
Broad Award Slate

Deferred Applications

Steve Clauser, PhD, MPA

Program Director,
Healthcare Delivery and Disparities Research

Cycle 1 2020 – Broad PFA Overview



Cycle 1 2020 – Broad Funding Slate

2 Recommended Additions to Slate

Previously
Approved
Slate:
8
Projects

Slate with
Proposed
Additions:
 $8 + 2 = 10$
Projects



Project Title

Biologic Abatement and Capturing Kids' Outcomes & Flare Frequency in Juvenile Spondyloarthritis (BACK OFF JSpA)

A Comparison of Prolonged Exposure Therapy, Pharmacotherapy, and their Combination for PTSD: What Works Best, and for Whom

A Real-World Comparative Effectiveness Trial of Treatment Strategies in Patients with Rheumatoid Arthritis: The RA-PRO PRagmatic trial (RA-PROPR)

Methods to Analyze Patient-centered Outcomes Missing due to Death in Cluster-randomized Trials

Teletherapy to Address Language Disparities in Deaf and Hard-of-Hearing Children

Social Interventions for Support during Treatment for Endometrial Cancer and Recurrence: SISTER study

Comparing the Effectiveness of Behavioral Health Supports for Publicly-Insured Perinatal Women

Helping Patients Achieve Kidney Transplants through Health System Change

Advancing Perinatal Mental Health and Wellbeing: The DC Mother-Infant Behavioral Wellness Program

Development of Methods to Improve Identification of Patients with Rare or Complex Diseases

Total Funds Requested*:

\$5.3

*Total Award with Proposed Addition to Slate: \$45.8M

Note: 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 addition of the recommended projects to the slate of awards from the Cycle 1 2020 Broad PFAs

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

Dissemination and Implementation Award Slates

Mike Herndon, DO

Chair, Engagement, Dissemination, and Implementation
Committee



Limited Competition: Dissemination and Implementation PFA

Implementation of PCORI-Funded Patient-
Centered Outcomes Research Results

Cycle 2 2020 Award Slate

Joanna Siegel, SM, ScD

Program Director, Dissemination and Implementation

Cycle 2 2020 - Limited Competition Dissemination and Implementation PFA

Slate of 3 Recommended Projects



Project Title	
Implementation of Health-E You/Salud iTu to Promote Adolescent-Centered Contraceptive Care	
Implementation of a Self-Care Plan for Patients with Acute Heart Failure Discharged from the ED	
Implementing Advance Care Planning for Dialysis Patients	
Total Funds Requested:	\$3.8M

* 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

Implementation of Effective Shared Decision Making Approaches in Practice Settings PFA

Cycle 2 2020 Award Slate

Joanna Siegel, SM, ScD

Program Director, Dissemination and Implementation

Cycle 2 2020 – Implementation of Effective Shared Decision Making Approaches in Practice Settings PFA

Slate of 1 Recommended Project



Project Title	
Taking ‘What Matters Most’ to Scale by Implementing a Breast Cancer Coproduction Learning Collaborative	
Total Funds Requested:	\$2.1M

* 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 slates:
 - Cycle 2 2020 Limited Competition Dissemination and Implementation PFA
 - Cycle 2 2020 Implementation of Effective Shared Decision Making Approaches in Practice Settings 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

Voice Vote:

- Vote to **Approve** the **Final** Motion
 - Ask for votes in favor, opposed, and abstentions

Overview: Developing Targeted PFAs in the Short-term while Strategic Planning is Underway

Nakela L. Cook, MD, MPH
Executive Director



Maintaining Pace on Our Commitment Plan



At our last meeting:

- The Board discussed our *expedited and enhanced approach* to developing Targeted PFAs

Today the Board considers:

- Approval of *three Targeted PFAs* recommended by SOC for Cycle 2 2021

Looking ahead to April:

- The Board will consider a *set of topics for development for future cycles*

Alignment with Research Agenda and Standard TPFA Development Process

- All topics leverage prior and ongoing work, have high likelihood of convergence with National Priorities and Research Agenda, and meet these criteria:
 - High burden conditions and issues
 - Mandates and/or expressed interest from Stakeholders
 - Relate to themes emerging from Strategic Planning
- Expedited development of targeted PFAs still includes:
 - Literature review
 - Outreach to relevant patient groups and stakeholders
 - Review of PCORI's funded portfolio
 - Consultation with NIH, AHRQ, and other relevant funders

Anticipated Advantages of Enhanced Approach to Targeted PFAs

Main differences in resulting Targeted PFAs:

- *More open for research and stakeholder communities to develop and propose relevant studies:*
 - Highly-specified study parameters when appropriate
 - Wider range of study duration and funding level
 - Greater total PFA funding to accommodate more and/or larger studies
- *Increased likelihood of accomplishing the objective of the PFA*
 - Offer more than one cycle as warranted
 - Allow for deferrals and resubmissions as needed

Summary of Topics Under Consideration Today for Cycle 2 2021



Topic	Funding
Improving Postpartum Maternal Outcomes for Populations Experiencing Disparities	Up to \$ 50M
Comparative Effectiveness of Interventions Targeting Mental Health Conditions in Individuals with Intellectual and Developmental Disabilities	Up to \$ 40M
Nonsurgical Interventions for Women with Urinary Incontinence	Up to \$ 40M
Total	Up to \$130M

Targeted PFAs Recommended for Cycle 2 2021

Alicia Fernandez, MD

Chair, Science Oversight Committee

Improving Postpartum Maternal Outcomes for Populations Experiencing Disparities

Els Houtsmuller, PhD

Associate Director,
Healthcare Delivery and Disparities Research

Recommendation for Targeted PFA

Question of interest:

What is the comparative effectiveness of multicomponent interventions to improve early detection of and timely care for risk factors for postpartum complications, and for complications during the first 6 weeks postpartum for Black, American Indian/Alaskan Native, and Hispanic women, women who live in rural areas, and women with low socioeconomic status (SES)?

Recommendation for Targeted PFA

Population	Populations at increased risk of postpartum maternal mortality, severe maternal morbidity and pregnancy complications, including Black, Hispanic, and American Indian/Alaskan Native women, women who live in rural areas, and women with low SES status
Intervention/ Comparators	Multicomponent interventions to increase awareness and detection of risk factors for postpartum complications (e.g., diabetes, hypertension, primiparity, body mass index, age) and postpartum complications (e.g., infection, hemorrhage, stroke, hypertensive disorders of pregnancy, cardiomyopathy)
Outcomes	Maternal morbidity and mortality; healthcare utilization; condition-specific outcomes; patient experience, satisfaction, and engagement with care
Timing	1 year postpartum

- Total requested for this PFA is up to \$50M in total costs
- Estimated number of cycles: 2-3
- Estimated number of studies: 4-6
- Maximum project duration: 5 years

Board Vote

Call for a Motion to:

- **Approve** the development of a PFA on “Improving Postpartum Maternal Outcomes for Populations Experiencing Disparities” with funding up to \$50M in total costs

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

Comparative Effectiveness of Interventions Targeting Mental Health Conditions in Individuals with Intellectual and Developmental Disabilities

Holly Ramsawh, PhD

Senior Program Officer,
Clinical Effectiveness and Decision Science

Recommendation for Targeted PFA

Question of interest:

What is the comparative effectiveness of evidence-based approaches (e.g., specific pharmacologic and behavioral interventions) that address mental health conditions in individuals with intellectual and developmental disabilities (IDD)?

Recommendation for Targeted PFA

Population	Individuals with both intellectual and developmental disabilities (IDD) and mental health conditions.
Intervention/Comparators	Head-to-head comparisons of evidence-based interventions that target mental health conditions in individuals with IDD.
Outcomes	Symptom severity, functional impairment, quality of life, adverse events, health resource utilization, relevant caregiver outcomes, and related outcomes.
Timing	Up to 12-month follow-up.

- Total requested for this PFA is up to \$40M in total costs
- Estimated number of cycles: 2-3
- Estimated number of studies: 10-12
- Maximum project duration: 3 years

Board Vote

Call for a Motion to:

- **Approve** the development of a PFA on “Comparative Effectiveness of Interventions Targeting Mental Health Conditions in Individuals with IDD” with funding up to \$40M in total costs

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

Nonsurgical Interventions for Women with Urinary Incontinence Targeted PFA

Nora McGhee, PhD

Senior Program Officer,
Clinical Effectiveness and Decision Science

Recommendation for Targeted PFA

Question of interest:

What is the comparative effectiveness of nonsurgical interventions for non-pregnant women with urinary incontinence?

Recommendation for Targeted PFA

Population	Women with stress, urge, or mixed urinary incontinence (UI). If more than one type of UI will be included, should be powered for subgroup analyses to allow conclusions for specific types.
Intervention/Comparators	Nonsurgical UI treatments and systems approaches
Outcomes	Functional outcomes and adverse events important to patients
Timing	Follow-up time to be determined based on appropriateness for the intervention/design

- Total requested for this PFA is up to \$40M in total costs
- Estimated number of cycles: 2
- Estimated number of studies: 5-8
- Maximum project duration: 5 years

Board Vote

Call for a Motion to:

- **Approve** the development of a PFA on “Nonsurgical Interventions for Women with Urinary Incontinence” with funding up to \$40M in total costs

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

Set of Candidate Topics for Targeted PFA Development for Future Cycles through FY2022

Nakela L. Cook, MD, MPH
Executive Director



Next Step in Expedited and Enhanced Approach to Developing Targeted PFAs

- Board considers *set of topics for development for future cycles*
 - Individual TPFAs will be recommended by SOC to Board for approval after further development
 - Set is *in addition to* COVID-19, MMM, IDD topics
- Programming *remains responsive to developments*
 - Within proposed topic area
 - With respect to emerging topics
 - Inclusive of learning from each PFA

Advantages of Programming a Set of Topics for the Next Several Funding Cycles

- *Expedited Targeted PFA development process:*
 - Allows longer-range planning
 - Focuses staff resources
- *Greater advance notice for research and stakeholder communities:*
 - Could increase readiness to submit applications
 - Might result in greater number of more fully developed applications

Approving PCORI's “Principles for the Consideration of the Full Range of Outcomes Data In PCORI-Funded Research”

Nakela L. Cook, MD, MPH

Executive Director

Andrew Hu, MPP

Director, Public Policy and Government Relations

Joanna Siegel, ScD

Program Director, Dissemination & Implementation

Overview of PCORI's 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:** Principles and Initial Guidance for Applicants **by March 2021**

Pillar 2

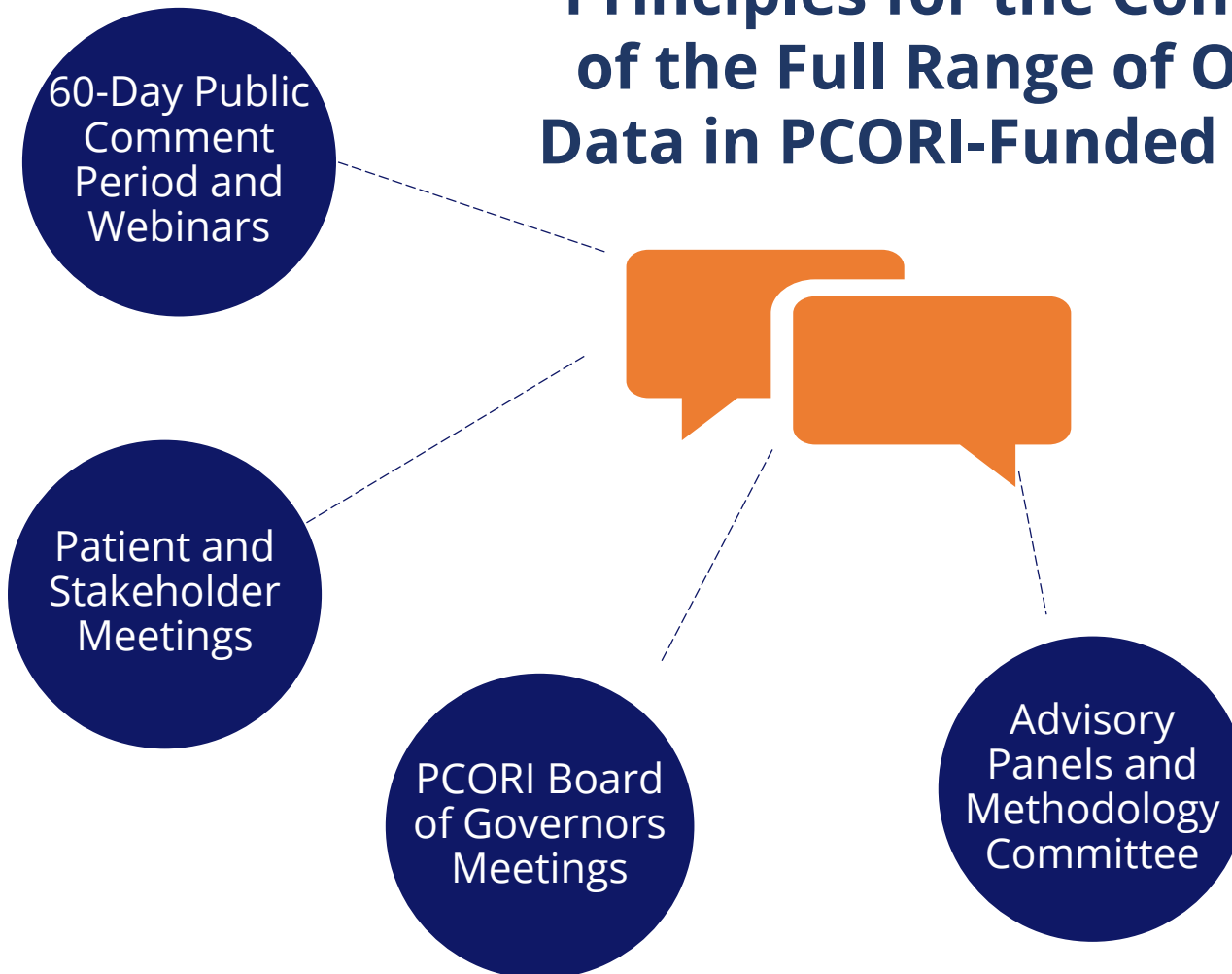
- Advancing scientific methods in relation to the collection and consideration of burden and economic impact data.
- **Timeline:** Approximately **12 months** from the initiation of this process

Pillar 3

- Supporting discussions related a patient-centered approach to addressing rising healthcare costs and value.
- **Timeline:** Ongoing Discussion

Process for Collection of Input on Principles

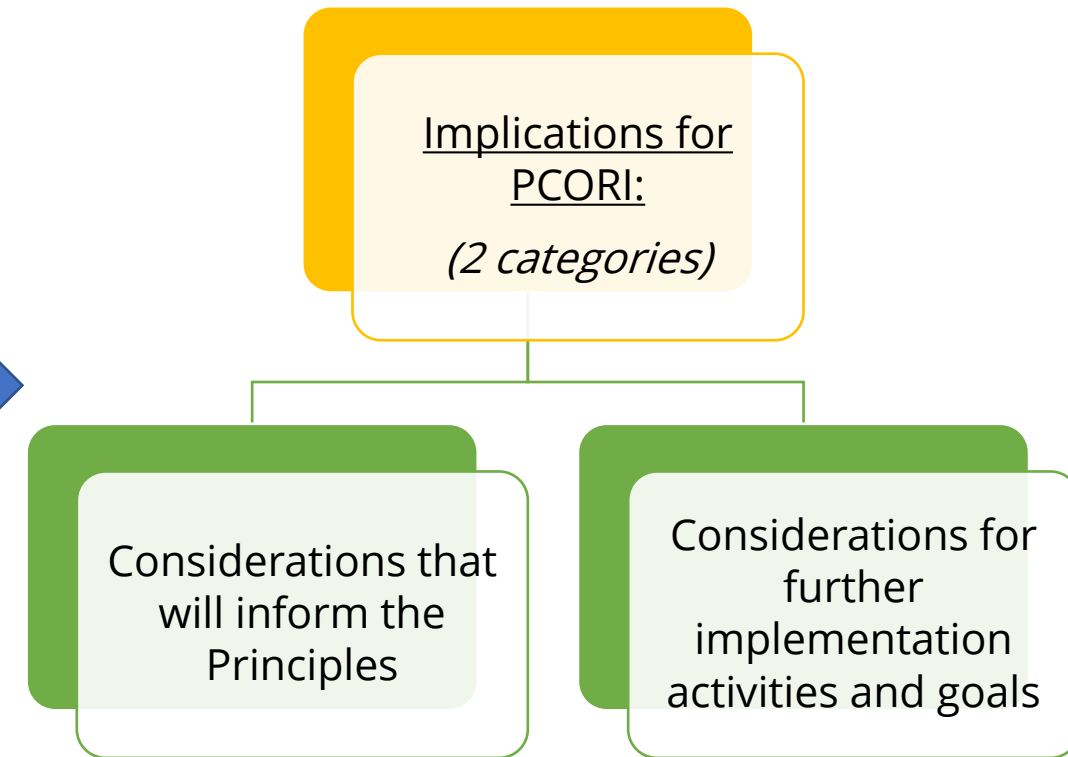
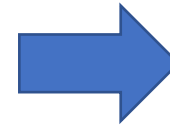
“Principles for the Consideration of the Full Range of Outcomes Data in PCORI-Funded Research”



Overarching Themes and Potential Considerations

What PCORI Heard

- Ensure Patient-Centeredness
- Conduct Ongoing Stakeholder Engagement
- Burden and Economic Data Should Be Collected When Appropriate, But **Not** Required
- Provide Additional Guidance to Reflect the New Authority
- Identify Ways for PCORI to Inform/Advance Rigor and Standards When Considering Burden and Economic Data
- Consider Social Risk Factors



Principle #1:

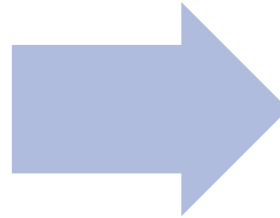
Identifying Outcomes Important to Patients and Caregivers



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

What PCORI Heard

- Maintain Principle 1 as central to PCORI's work
- Ensure patient and caregiver engagement
- Prioritize measures important to patients and caregivers
- Address unintended consequences



How We Revised the Proposed Principles

- Emphasized that patient-centricity is central to PCORI-funded research
- Noted importance of engagement to identify the relevant outcomes
- Clarified that the full range of outcomes still include health and patient-reported outcomes

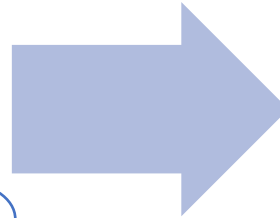
Principle #2:

Identifying Outcomes Important to Stakeholders

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

What PCORI Heard

- Facilitate “continuous engagement” of diverse stakeholders
- Providing opportunities for engagement at the level of individual projects



How We Revised the Proposed Principles

- Clarified examples of outcomes and studies important to patients, caregivers and stakeholders

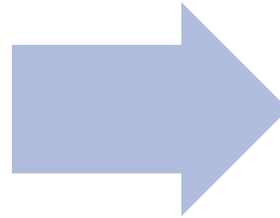
Principle #3:

Collection of Data as Appropriate and Relevant

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

What PCORI Heard

- Support for the collection of cost/economic impact data, but **not** requiring it
- Need for clarity on priority outcomes
- Important for outcomes to be appropriate and relevant
- Emphasis on the need to consider social risk factors



How We Revised the Proposed Principles

- Clarified that PCORI encourages, but will not require collection of this data
- Provided examples where the collection of data is appropriate and relevant
- Noted importance of engagement to identify appropriate and relevant outcomes
- Included considering social risk factors as an area of interest

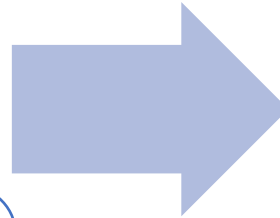
Principle #4:

Conduct of Certain Types of Economic Analysis

Principle #4: PCORI may support the conduct of certain types of economic analyses, as part of a funded research study or independently, to enhance the relevance and value to patients and other stakeholders of information PCORI-funded investigators collect on potential burdens and economic impacts.

What PCORI Heard

- Need for clarity on what types of analyses are allowable (provide examples)
- Clarify prohibition on developing or employing a dollars-per-quality adjusted life year threshold
- Maintain policy of not funding cost-effectiveness analysis



How We Revised the Proposed Principles

- Provided examples of allowable and prohibited analyses
- Clarified the prohibition on developing or employing a dollars-per-quality adjusted life year threshold for research funded by PCORI
- Maintained PCORI's policy of not funding cost-effectiveness analysis

Additional Implementation Activities

In addition to comments on the Principles, PCORI received input outlining specific areas for the focus of the Institute's ongoing activities to implement this authority. Below are additional implementation activities PCORI can undertake.

Ensuring Patient-Centeredness

Consider appropriate options to monitor how PCORI-funded research is used

Build on and develop additional toolkits to support patient and caregiver engagement

Advance and support discussions related to patient-centered approaches to rising healthcare costs and value

Ensuring Ongoing Patient and Stakeholder Engagement

Convenings to support direct engagement of stakeholders and researchers

Developing Guidance and Methodology Standards

Develop additional guidance to inform applicants

Support Methodology Committee activities to advance scientific rigor

Conduct ongoing review and evaluation of Principles, guidance, and methods

Principles for the Consideration of the Full Range of Outcomes Data in PCORI-Funded Research



Identifying Outcomes Important to Patients

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

Identifying Outcomes Important to Stakeholders

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

Criteria for Data Collection Opportunities

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

Consideration of Economic Analysis

- **Principle #4:** PCORI may support the conduct of certain types of economic analyses, as part of a funded research study or independently, to enhance the relevance and value to patients and other stakeholders of information PCORI-funded investigators collect on potential burdens and economic impacts.

Board Vote

Call for a Motion to:

- **Approve** PCORI's "Principles for the Consideration of the Full Range of Outcomes Data In PCORI-Funded Research"

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

Wrap Up and Adjournment



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