Board of Governors Meeting
via Teleconference/Webinar

May 8, 2017

10:00 am - 3:30 pm ET

pcori®

PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

Welcome and Introductions

Grayson Norquist, MD, MSPH
Chairperson, Board of Governors

Joe Selby, MD, MPH
Executive Director

Agenda

Time	Agenda Item
10:00-10:15	Welcome, Call to Order and Roll Call **Consider for Approval:** Consent Agenda • Minutes of March 21, 2017 Board Meeting • Revisions to Committee and Advisory Panel Charters
10:15-11:45	Joint Methodology Committee and Board Session: PCORI's Role in Evaluating Precision Medicine Treatments
11:45-12:15	**Consider Methodology Committee Report**: Recommendation to **Adopt** Updated Methodology Standards and **Accept** Revised Methodology Report
12:15-1:15	Break
1:15-1:45	Executive Director's Report and Q2 Dashboard Review
1:45-2:30	**Consider for Approval:** Targeted PFA Development • Medication Assisted Treatment (MAT) Delivery for Pregnant Women with Substance Use Disorders involving Prescription Opioids and/or Heroin • Symptom Management for Patients with Advanced Illness
2:30-3:00	Mid-Year Financial Review
3:00-3:30	Public Comment Period
3:30	Wrap-up and Adjourn Meeting of the Board

Consent Agenda Items

Grayson Norquist, MD, MSPH

Chairperson, Board of Governors

Joe Selby, MD, MPH

Executive Director

Motion for Consent Agenda Items

That the Board approve:

- Minutes from March 21, 2017 Board meeting
- Proposed amended Charters for the following PCORI Committees:
  - Research Transformation Committee (RTC)
  - Science Oversight Committee (SOC)
  - Engagement, Dissemination, and Implementation Committee (EDIC)
  - Governance Committee

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

Joint Methodology Committee and Board of Governors Session:

PCORI's Role in Evaluating Precision Medicine Treatments

Welcome & Session Overview

Joe Selby, MD. MPH

Executive Director

Defining a PCORI Research Agenda in Precision Medicine

Evelyn P. Whitlock, MD, MPH

Chief Science Officer

What is Meant by Precision Medicine?

- NIH: Precision medicine is “an emerging approach for disease treatment and prevention that takes into account individual variability in genes, environment, and lifestyle for each person”
- Various formulations may reflect a broader, narrower or slightly different perspective
  - Precision-Directed Initiatives, NEJM, April 27, 2017 (http://www.nejm.org/doi/full/10.1056/NEJMp1613224)
  - Personalized medicine
  - Genomic medicine
- Today’s discussion is focused on exploring the range and focus of PCORI’s current research agenda in “Precision Medicine”

Challenges for CER in Precision Medicine Activities

- Precision medicine is a relatively young field, early in its development, with rapidly changing knowledge in genetics, genomics, and molecular diagnostics
- Evidence from primarily early phase studies presents challenges for CER
  - CER requires efficacious or widely available comparators
  - Lack of insurance coverage for many new technologies
  - Durability and timeliness of evidence
- Nonetheless, these technologies are rapidly diffusing and stakeholders are interested in whether and how these developments contribute to patient-centered care

Opportunities for PCORI in Precision Medicine

- Precision medicine is inherently patient-centered and aligned with PCORI's goal to determine "what works best for whom under what circumstances"
  - PCORI supports methodological research to advance understanding and appropriate use of methods for ascertaining variation in treatment effects (benefits and harms)
  - PCORI can conduct critical research incorporating patient perspectives on how best to communicate and support decision-making for genomic and non-genomic targeted treatments
  - Surveillance and partnerships (e.g., NIH, FDA) can identify focused topics ready for patient-centered CER
  - Monitoring diffusion of precision medicine technologies, perhaps through PCORnet, can identify researchable targets in emerging issues (i.e., whether "better outcomes" are seen with genomically-targeted treatments in cancer or not)

PCORI is an Important Contributor

- While NIH and others are committing substantial resources to Precision Medicine, PCORI can contribute in ways to complement and extend others' investment
- Having a clearer framework for the scope and focus of PCORI's current research agenda in Precision Medicine will enable us to more effectively identify areas for focused funding and to respond to investigator-initiated ideas
- Given the rapid evolution of the field, the research agenda will deserve revisiting and revision at least every few years

Panel Presentations: Methodology Committee Perspectives

Robin Newhouse, PhD, RN

Chair, PCORI Methodology Committee

Steven Goodman, MD, MHS, PhD

Vice Chair, PCORI Methodology Committee

Naomi Aronson, PhD

PCORI Methodology Committee

Discussion Questions

Considering both the predominant focus on molecular diagnostics and targeted therapies, as well as a broader approach to targeting prevention and treatment approaches based on individual characteristics…

- What are the opportunities for PCORI to make value-added contribution(s) to the field of precision medicine?
- What are the challenges for PCORI?
- What is needed to support the success of these efforts?

Methodology Committee Report

Robin Newhouse, PhD, RN

Chair, PCORI Methodology Committee

Steven Goodman, MD, MHS, PhD

Vice Chair, PCORI Methodology Committee

Methodology Committee Members

- Robin Newhouse, Chair
- Steven Goodman, Vice Chair
- Naomi Aronson
- Ethan Basch
- Stephanie Chang
- David Flum
- Cindy Girman
- Mark Helfand
- Michael Lauer
- David Meltzer
- Brian Mittman
- Sally Morton
- Neil Powe
- Adam Wilcox
- Susan Zickmund (Advisor)

PCORI's Methodology Standards

- Required by the authorizing legislation
- Represent minimal standards for design, conduct, and reporting of comparative effectiveness research (CER) and patient-centered outcomes research (PCOR)
- Provide guidance to researchers and those who use research results
- Reflect generally accepted best practices
- Used to assess the scientific rigor of funding applications and monitor conduct of research awards

Methodology Standards Update: Process

- Applied a systematic process to review, revise, and update the original 47 Methodology Standards (adopted in 2013)
- Created a new category of Methodology Standards on research designs using clusters
- Posted the updated Methodology Standards for public comment (February – April 2017)
- Updated Methodology Standards based on public comments received
- Methodology Committee approved updates (March 20, 2017)

Methodology Standards Update: Overview

Summary of Changes:

- 48 standards in 12 categories
- 1 new cross-cutting standard under the Standards for Causal Inference Methods (CI-1: Specify the causal model underlying the research question)
- 5 new standards for research designs using clusters

General rationales for the updates are to:

- Streamline and clarify language
- Ensure alignment of the standards
- Reflect advances in methods for PCOR/CER

Methodology Standards Update: Highlights

- **Research Question-2: Develop a formal study protocol**
  - Required elements of a study protocol and documentation of amendments
- **Patient Centeredness-4: Support dissemination and implementation of study results**
  - Study results be made publically available and presented in lay language summaries
- **Data Networks-1: Requirements for the design and features of data networks**
  - Additional expectations for privacy protections
- **Data Networks-2: Selection and use of data networks**
  - Ensure appropriateness of data in the network(s) for a specific research question
- **Standards for Studies of Medical Tests (MT-1, MT-2, MT-3)**
  - Applicable to studies of any test used to inform medical decision making (previously “Standards for Studies of Diagnostic Tests”)

Methodology Report Revisions: Overview

Systematic process to review and update the Methodology Report to:

- Reflect advances in methods and related efforts in PCOR/CER
- Update references to the scientific literature
- Incorporate additional public comments
- Improve clarity in the general guidance and discussion

Summary of Changes

- Sections I-IV were updated and streamlined
- Background/rationale sections (in Section III) were updated to reflect the updated standards

Next Steps

- Recommending adoption of the updated Methodology Standards today
- Implementing the updated Methodology Standards
  - Cycle 2 2017 funding cycle (applications due 10/25/2017)
- Updating training and resources for use of the Methodology Standards
- Continuing work on the Methodology Standards and Methodology Report
  - Development of additional standards (FY17 Priorities)
    - Complex Interventions
    - Data Management
    - Data Quality
    - Individual participant data (IPD) and network meta-analysis
    - Qualitative Methods

Board Vote

Call for a Motion to:	• **Adopt** the updated PCORI Methodology Standards and **Accept** the Revised Methodology Report
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

Break

We will return at 1:15 pm ET

Join the conversation on Twitter via @PCORI

Executive Director's Report & Q2-2017 Dashboard Review

Joe Selby, MD, MPH

Executive Director

PCORI's Era of Results

PCORI Priority Area	# of Articles
Clinical Effectiveness / Communication & Dissemination Research	23
Addressing Disparities/ Improving Healthcare Systems	4
Total	**27**

27

Publications with CER Results

In High Impact Journals

- JAMA (3)
- BMJ (3)
- Annals of Internal Medicine (1)

Results Focus on a Range of Topics

Clinical Effectiveness/ Communication & Dissemination Research

Cardiovascular Health

Prostate Cancer

Serious Bacterial Infection in Children

Addressing Disparities/ Improving Healthcare Systems

Peer Health Navigation in Mental Health

Family-Reported Errors & Adverse Events

Reducing High-Dose Chronic Opioid Therapy

2017 Annual Meeting
Delivering Results, Informing Choices

October 31 – November 2

Crystal Gateway Marriott
Arlington, VA

Agenda Sessions

Four plenary and **eight breakout sessions featuring research results**, workshops, poster session, and networking opportunities.

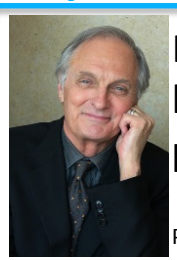
Attendees

1,000 – 1,300 members of the PCORI Community including researchers, patients & caregivers, clinicians, employers, insurers, staff & others. Plus another 500+ on webcasts.

Scholarships

100+ scholarships for patients/caregivers & 100 for health research trainees.

Key Note Speakers



Keynote speakers include **Alan Alda**, long-time advocate for improving public understanding of science.

Photo Credit: The Alda Office

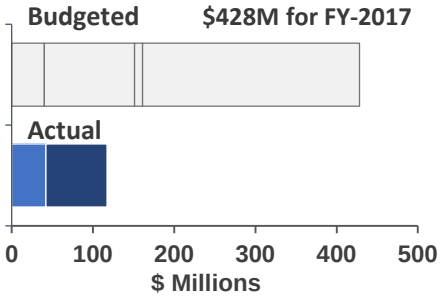
Board of Governors Dashboard
Second Quarter FY-2017 (As of 3/31/2016)

Our Goals: Increase Information, Speed Implementation, and Influence Research

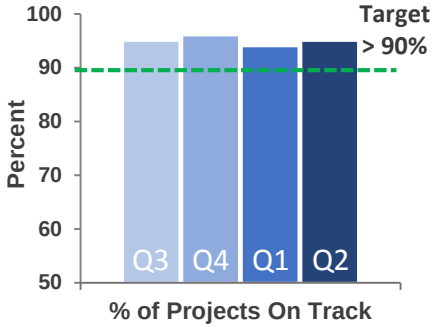
On Target | Off Target | Needs Board Attention | Too Early to Evaluate / No Target | Projected/Target | Q3 2016 | Q4 2016 | Q1 2017 | Q2 2017

Funds Committed to Research

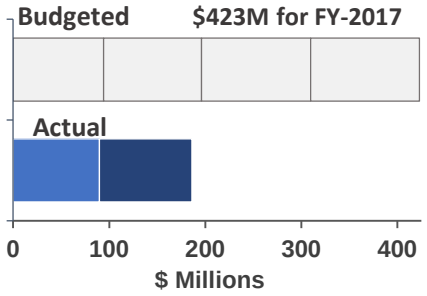
Includes funds committed to PCORnet



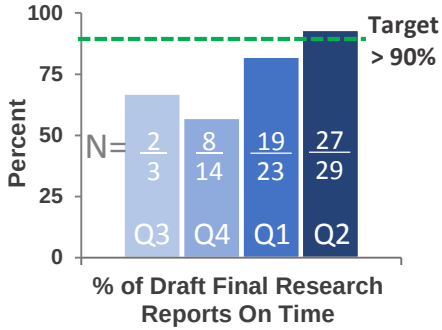
Project Performance



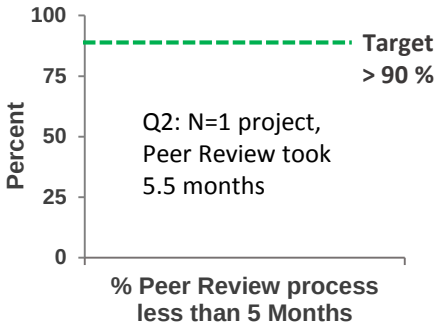
Budget



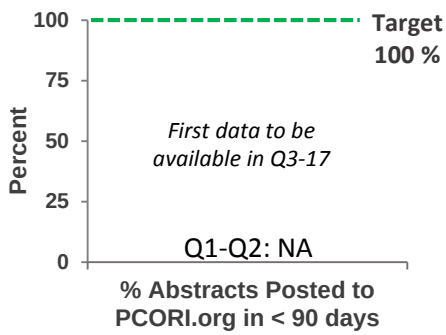
Draft Final Research Reports



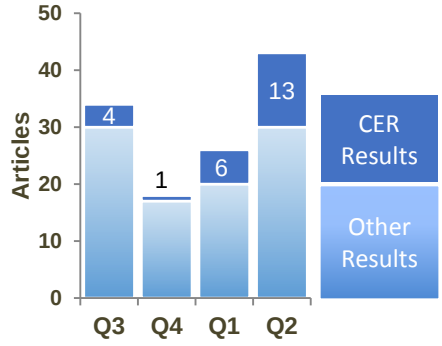
PCORI Peer Review



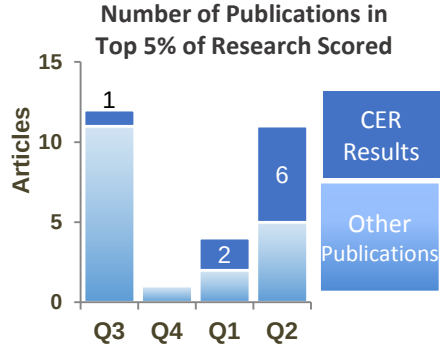
Public Release of Research Findings



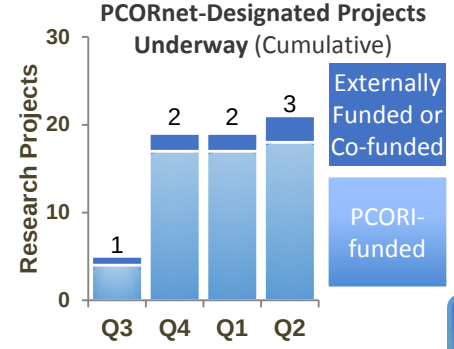
Results Published in Literature



Altmetrics



Research in PCORnet



Inputs | Process | Outputs | Uptake | Use | Impact

Narrative Examples

Increasing Information

Two PCORI studies of prostate cancer treatment published results in JAMA in March 2017, showing the adverse effects and quality of life as reported by men with localized prostate cancer who chose treatment, observation, or active surveillance. The information provided by these studies may facilitate patient counseling regarding the expected harms of contemporary treatments and their possible effect on quality of life.

Increasing Information

A PCORI-funded study of stroke prevention therapies found that among patients with atrial fibrillation who had experienced an acute ischemic stroke, inadequate therapeutic anticoagulation preceding the stroke was prevalent.

Influencing Research

The Editorial Board of the journal "SLEEP" credits PCORI with their new editorial guidance to authors requiring the use of people-centered language in all publications.

Results of PCORI-Funded Research:
Helping Men with Prostate Cancer Make Better-Informed Choices Among Modern-day Treatments

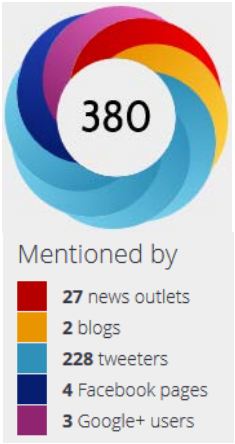
Two PCORI studies of prostate cancer treatment published results in JAMA in March 2017, showing the adverse effects and quality of life as reported by men with localized prostate cancer who chose treatment, observation, or active surveillance.

> This information may facilitate patient counseling regarding the expected harms of contemporary treatments and their possible effect on quality of life.
>
> *-Barocas et al.*

Barocas DA, et al. **Association Between Radiation Therapy, Surgery, or Observation for Localized Prostate Cancer and Patient-Reported Outcomes After 3 Years**. *JAMA*. Mar 2017.

Men who had their prostate cancer treated with either the latest forms of surgery or radiation therapy experienced greater rates of problems, either urinary, bowel or sexual, during the first several months compared to those who opted for active surveillance. However, outcomes among all groups were similar after two years.

- Principal Investigator: David Penson, MD, MPH, Vanderbilt University



Chen RC, et al. **Association Between Choice of Radical Prostatectomy, External Beam Radiotherapy, Brachytherapy, or Active Surveillance and Patient-Reported Quality of Life Among Men With Localized Prostate Cancer.** *JAMA*. Mar 2017.

Surgery to treat prostate cancer, including robot-assisted procedures, was associated with greater rates of incontinence and sexual problems than either external beam radiation or watchful waiting even three years afterward. But surgery also resulted in fewer other urinary symptoms than active surveillance.

- Principal Investigator: Ronald Chen, MD, MPH, University of North Carolina at Chapel Hill

Results of PCORI-Funded Research:

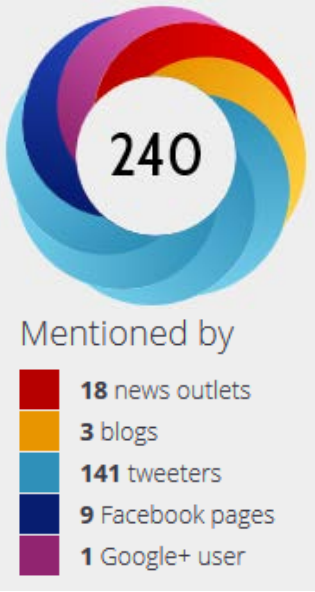
In Atrial Fibrillation, Preceding Antithrombotic Treatment is Associated with Lower Severity of and In-Hospital Mortality from Acute Ischemic Stroke

Xian Y, et al. **Association of Preceding Antithrombotic Treatment With Acute Ischemic Stroke Severity and In-Hospital Outcomes Among Patients With Atrial Fibrillation**. *JAMA*. Mar 2017.

Study title: Patient-Centered Research into Outcomes Stroke Patients Prefer and Effectiveness Research (PROSPER)

Antithrombotic therapies are known to prevent stroke for patients with atrial fibrillation (AF) but are often underused. This retrospective observational study of 94,474 patients with acute ischemic stroke and known history of AF found ***that 84% did not receive guideline-recommended therapeutic anticoagulation, and 30% of patients were not receiving any antithrombotic treatment***. Some of these patients had reasons for not being anticoagulated, such as high bleeding or fall risk, but more than 2/3 had no documented reason for receiving inadequate stroke prevention therapy.

After adjusting for potential confounders, ***compared with no antithrombotic treatment, preceding use of therapeutic warfarin, NOACs, or antiplatelet therapy was associated with lower odds of moderate or severe stroke and in-hospital mortality***.



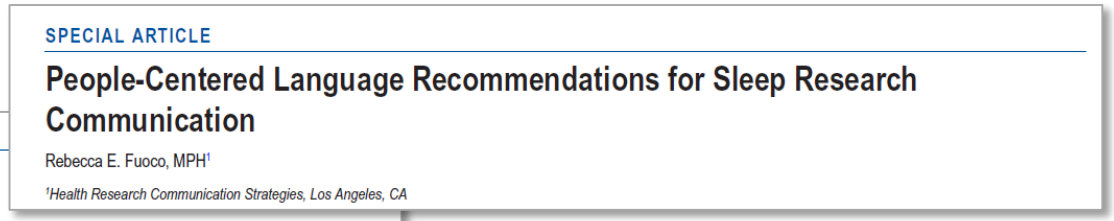
- Principal Investigator: Adrian Hernandez, MD, MHS, Duke University

> We estimate that between 58,000 to 88,000 strokes might be preventable per year if the treatment guidelines are followed appropriately.
>
> *-Dr. Ying Xian, MD MPH, Study Co-Investigator*
>
> *MedicalResearch.com Article*

Goal 3: Influencing Research Example:

Increasing Patient-Centeredness in Scientific Publications

At a conference supported by a PCORI Eugene Washington Engagement award (Lead: Parthasarathy), a presentation by Rebecca Fuoco, MPH, communication specialist (Project Sleep - - the patient advocacy partner), inspired the Editorial Board of the journal "SLEEP" to ***issue new editorial guidance to authors requiring the use of people-centered language in all publications***.

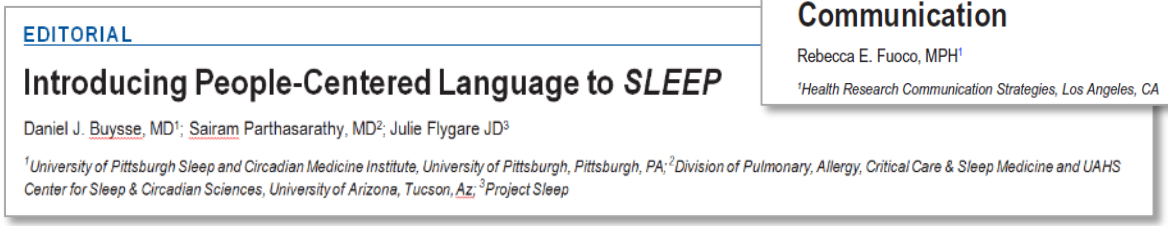


SPECIAL ARTICLE

People-Centered Language Recommendations for Sleep Research Communication

Rebecca E. Fuoco, MPH[1]

[1]Health Research Communication Strategies, Los Angeles, CA



EDITORIAL

Introducing People-Centered Language to *SLEEP*

Daniel J. Buysse, MD[1]; Sairam Parthasarathy, MD[2]; Julie Flygare JD[3]

[1]University of Pittsburgh Sleep and Circadian Medicine Institute, University of Pittsburgh, Pittsburgh, PA; [2]Division of Pulmonary, Allergy, Critical Care & Sleep Medicine and UAHS Center for Sleep & Circadian Sciences, University of Arizona, Tucson, Az; [3]Project Sleep

> Ms. Fuoco's presentation on people-centered language led to the obvious conclusion that the field's flagship journal should follow best practices.
>
> *- Buysse, Parthasarathy, & Flygare, 2017*

> The establishment of the Patient-Centered Outcomes Research Institute ***(PCORI) in 2010 ushered in a new era of patient – professional partnerships in medical research*** and health care… PCORI has made overt what was actually true all along: That meaningful medical research is by definition a collaborative effort.
>
> *- Buysse, Parthasarathy, & Flygare, 2017*

High Altmetric Scores

Publications from PCORI-Funded Research, Q2-17

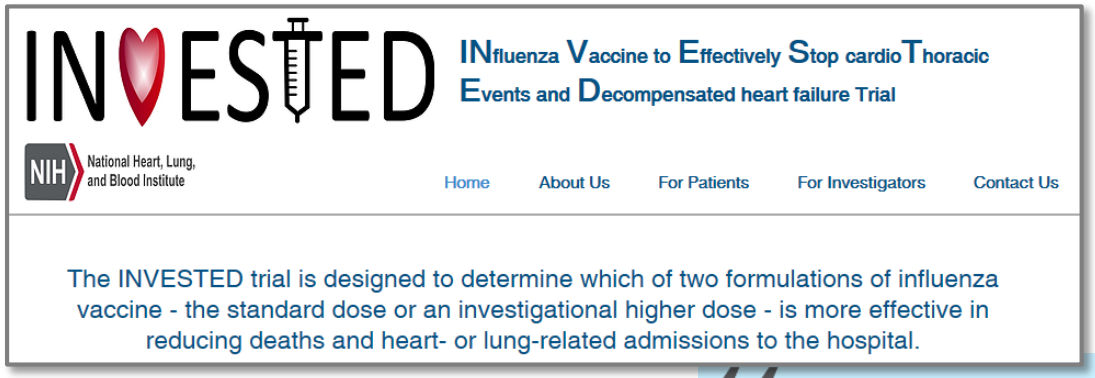
11 publications from Q2-17 have high Altmetric scores, and 6 of these publications (listed below) have very high Altmetric scores (>100). The score indicates attention in news articles (red), on social media (blues), and in blogs (gold).

Altmetric	Publication
	Barocas DA, et al. **Association Between Radiation Therapy, Surgery, or Observation for Localized Prostate Cancer and Patient-Reported Outcomes After 3 Years**. *JAMA*. Mar 2017. CER RESULT
	Chen RC, et al. **Association Between Choice of Radical Prostatectomy, External Beam Radiotherapy, Brachytherapy, or Active Surveillance and Patient-Reported Quality of Life Among Men With Localized Prostate Cancer.** *JAMA*. Mar 2017. CER RESULT
	Falade-Nwulia O,et al**. Oral Direct-Acting Agent Therapy for Hepatitis C Virus Infection: A Systematic Review**. *Ann Intern Med.* Mar 2017. CER RESULT
	Khan A, et al. **Families as Partners in Hospital Error and Adverse Event Surveillance.** *JAMA Pediatr.* Feb 2017. CER RESULT
	Shen E, et al. **Association of a Dedicated Post-Hospital Discharge Follow-up Visit and 30-Day Readmission Risk in a Medicare Advantage Population**. *JAMA Intern Med*. Jan 2017. RESEARCH LETTER
	Xian Y, et al. **Association of Preceding Antithrombotic Treatment With Acute Ischemic Stroke Severity and In-Hospital Outcomes Among Patients With Atrial Fibrillation**. *JAMA*. Mar 2017. CER RESULT

PCORnet: Externally-Funded Projects

First Externally-Funded PCORnet-Designated Study – **the INVESTED Trial**

A comparative effectiveness study of doses of ***influenza vaccine among patients with a history of myocardial infarction or heart failure***. Funded by the NIH, this study seeks to enroll and randomize 9,300 patients, and will leverage 7 Clinical Data Research Networks (CDRNs).



"Early results from the INVESTED pilot study showing that the SMART IRB approach yielded an efficiency boost during startup foreshadows the improvements we anticipate as we implement the SMART IRB platform across all of our PCORnet studies. We hope to be able to share more positive results as we continue to analyze and collect more data from INVESTED."

Amy Franklin
PCORnet SMART IRB Coordinator

PCORnet: Externally-Funded Projects

AHRQ-funded Child Health Quality (CHeQ) Partnership

Department of Health Outcomes and Policy, University of Florida
Department of Pediatrics, University of Florida
Children's Hospital of Philadelphia (PEDSnet)

This PCORnet-Designated research project is a partnership between two dental plans, a CDRN, CHIP and Medicaid administrators, and managed care organizations. The collaborative team identified **dissemination and implementation opportunities for two Pediatric Quality Measurement Program-Centers of Excellence measure sets:**

- Linkage Between Dental Treatment and Dental Prevention
- Safe and Judicious Antipsychotic Use in Children and Adolescents

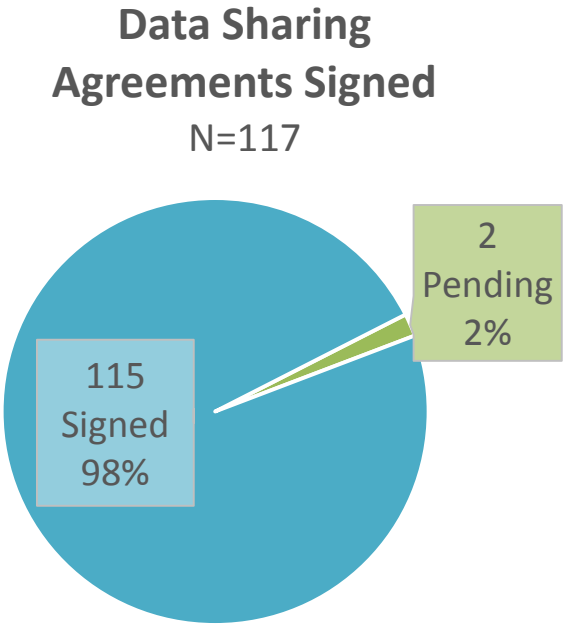
The goals of this project are to:

1. ***Test the feasibility and usability of these two measure sets*** (through field testing, refining, collecting data, and reporting on measures)
2. ***Translate the results into QI goals*** and multi-level performance improvement projects

PCORnet: Progress on Data Sharing Agreements

Data Sharing Agreements are signed by individual Network Data Affiliates. This agreement defines the standard terms to which the PCORnet Coordinating Center will adhere when information are sent from the affiliates to the Coordinating Center

As of Q2-2017, 98% of Data Sharing Agreements have been signed.



2017 Target

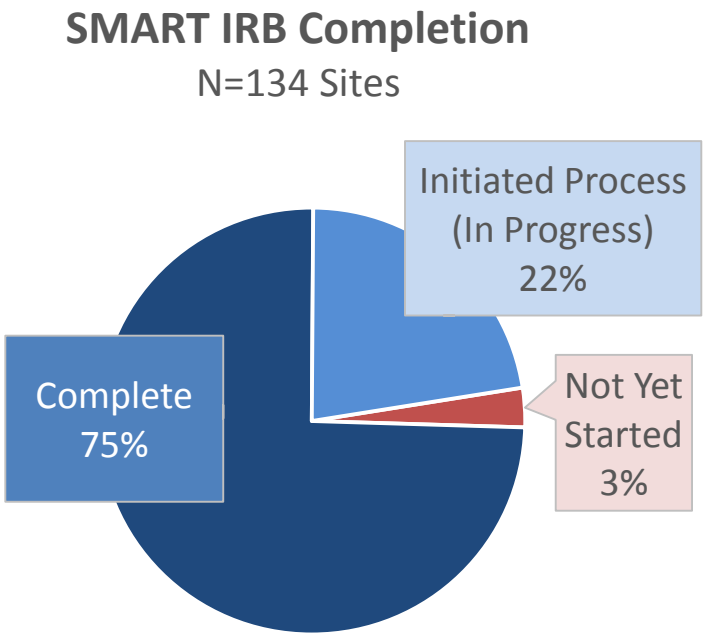
100% of sites will sign by June 1, 2017

Out of 117 affiliates, only two have not yet signed the Data Sharing Agreement (they were recent additions)

PCORnet: Progress on SMART IRB

The universal IRB form, called a SMART IRB, is used by all sites that make up CDRNs. SMART IRBs allow for the streamlining of clinical trials, reducing cost, and increasing efficiency and speed.

As of Q2-2017, 75% of sites within the CDRNs have completed the Smart IRB process



2017 Target

100% of sites will sign by May 25, 2017

- Of 134 sites, 100 have completed the SMART IRB, and 30 have initiated the process.
- 11 out of 13 CDRNs are 100% complete

PCORnet: PCORnet's Front Door is Open!

Request for Feasibility Review & Designation

Data Network Request

Request for Network Collaborators

- Front Door opened to the Network members in October 2016
- ***PCORnet was opened to public queries in April 2017***
- 65 total requests have been submitted through the Front Door (including internal and external queries)
- Events are being convened throughout the year with ***key audiences*** to highlight the opportunities to utilize PCORnet, starting with the 2017 ***Health Datapalooza*** conference sponsored by AcademyHealth held in April

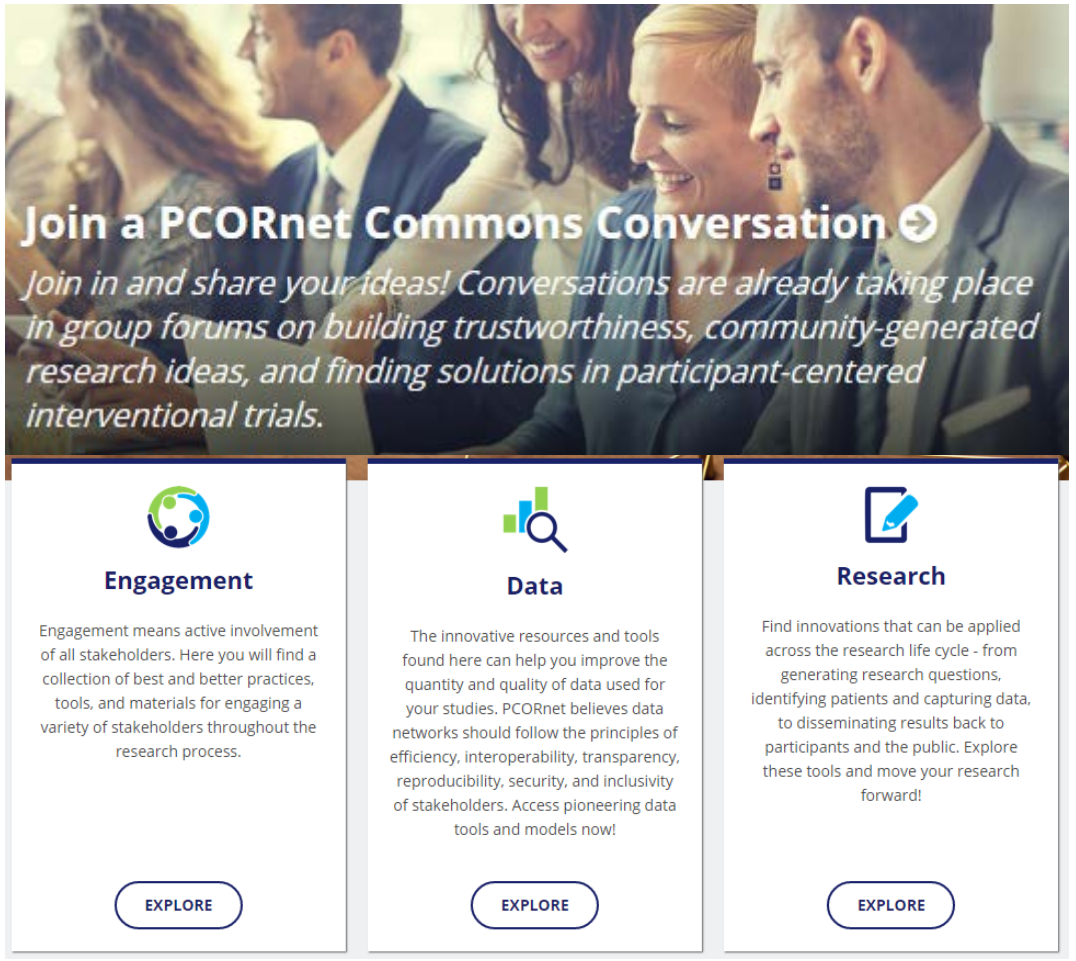
PCORnet

Enhancing Collaboration in PCORnet Commons

pcornetcommons

Increasing collaboration, efficiency, and people-centeredness in clinical research.

- ***Public platform*** for people interested in health research to collaborate, share and learn
- Launched in December 2016
- 21 collaborative groups including groups for ADAPTABLE, Demonstration Projects, and Collaborative Research Groups
- Continuous curation and development of resources
- Visit pcornetcommons.org



People-Centered Research Foundation (PCRF)

People-Centered
RESEARCH FOUNDATION

People-Centered Research Foundation

Launched March 21st, 2017

Mission: To *engage* *patients, families, research participants, clinicians, scientists, and health system leaders in the design, conduct, dissemination, and implementation of research and analysis that leads to improvements in the health and well-being of individuals and populations and the performance of health care delivery systems*

Timeline

- March, 2017:
  - Board members selected
  - Incorporated
- Spring, 2017:
  - Launch PCRF Program Office
  - Business development
- Summer, 2017
  - Contracts with Networks

Winter 2016-Early 2017
PCRF Governance Model and operationalization plan

March 2017
PCRF
Core Board established

PCRF

April 2017
Base operations Funding

June-July 2017
Business development
Contracts with networks executed

Summer/Fall 2017
Launch and full operations

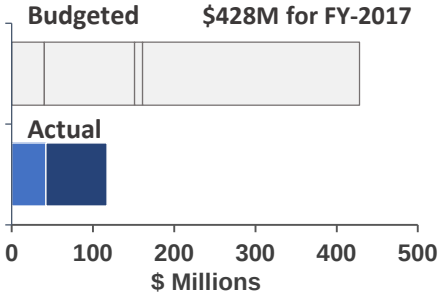
Board of Governors Dashboard
Second Quarter FY-2017 (As of 3/31/2016)

Our Goals: Increase Information, Speed Implementation, and Influence Research

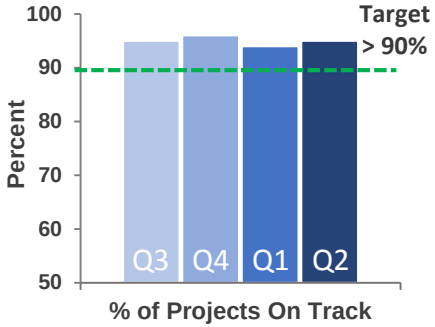
On Target
Off Target
Needs Board Attention
Too Early to Evaluate / No Target
Projected/Target
Q3 2016
Q4 2016
Q1 2017
Q2 2017

Funds Committed to Research

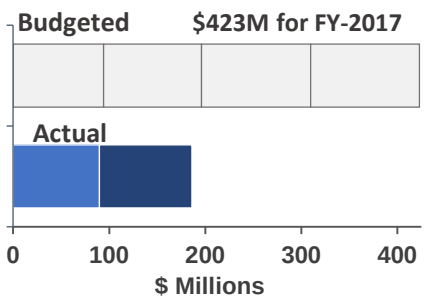
Includes funds committed to PCORnet



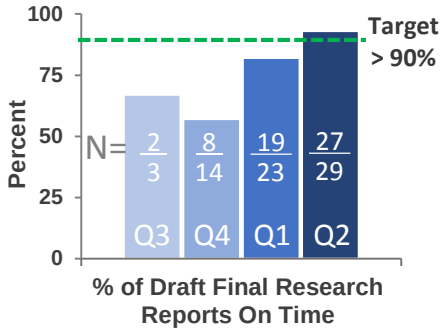
Project Performance



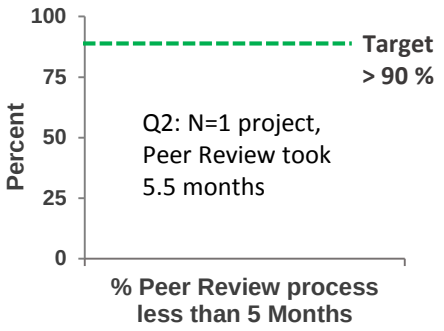
Budget



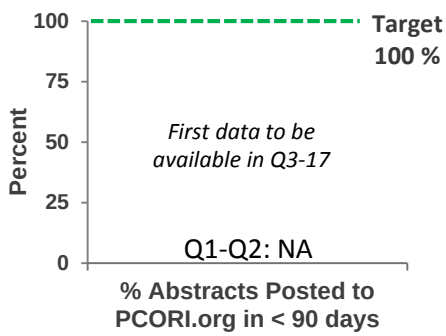
Draft Final Research Reports



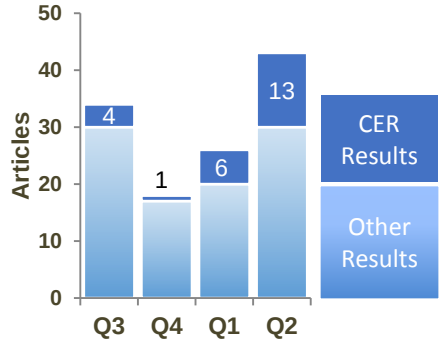
PCORI Peer Review



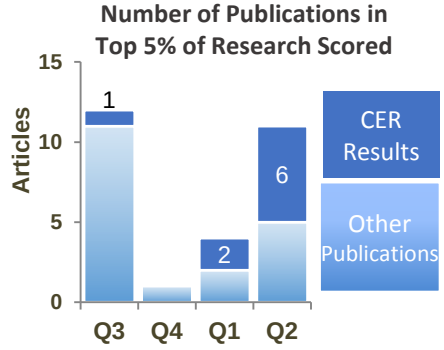
Public Release of Research Findings



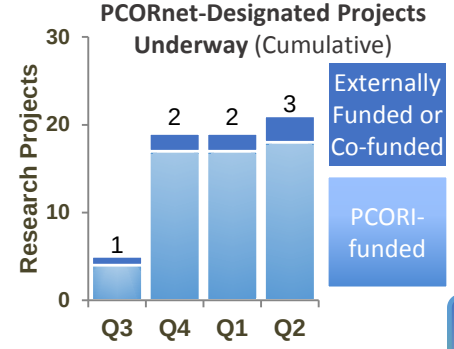
Results Published in Literature



Altmetrics



Research in PCORnet



Inputs
Process
Outputs
Uptake
Use
Impact

Narrative Examples

Increasing Information

Two PCORI studies of prostate cancer treatment published results in JAMA in March 2017, showing the adverse effects and quality of life as reported by men with localized prostate cancer who chose treatment, observation, or active surveillance. The information provided by these studies may facilitate patient counseling regarding the expected harms of contemporary treatments and their possible effect on quality of life.

Increasing Information

A PCORI-funded study of stroke prevention therapies found that among patients with atrial fibrillation who had experienced an acute ischemic stroke, inadequate therapeutic anticoagulation preceding the stroke was prevalent.

Influencing Research

The Editorial Board of the journal "SLEEP" credits PCORI with their new editorial guidance to authors requiring the use of people-centered language in all publications.

Medication-Assisted Treatment Delivery for Pregnant Women with Substance Use Disorders involving Prescription Opioids and/or Heroin

Recommendation for Targeted PFA Development Approval

Robert Zwolak, MD, PhD

Chair, Science Oversight Committee

Evelyn P. Whitlock, MD, MPH

Chief Science Officer

Current PCORI Funding Focused on Opioid Use/Addiction

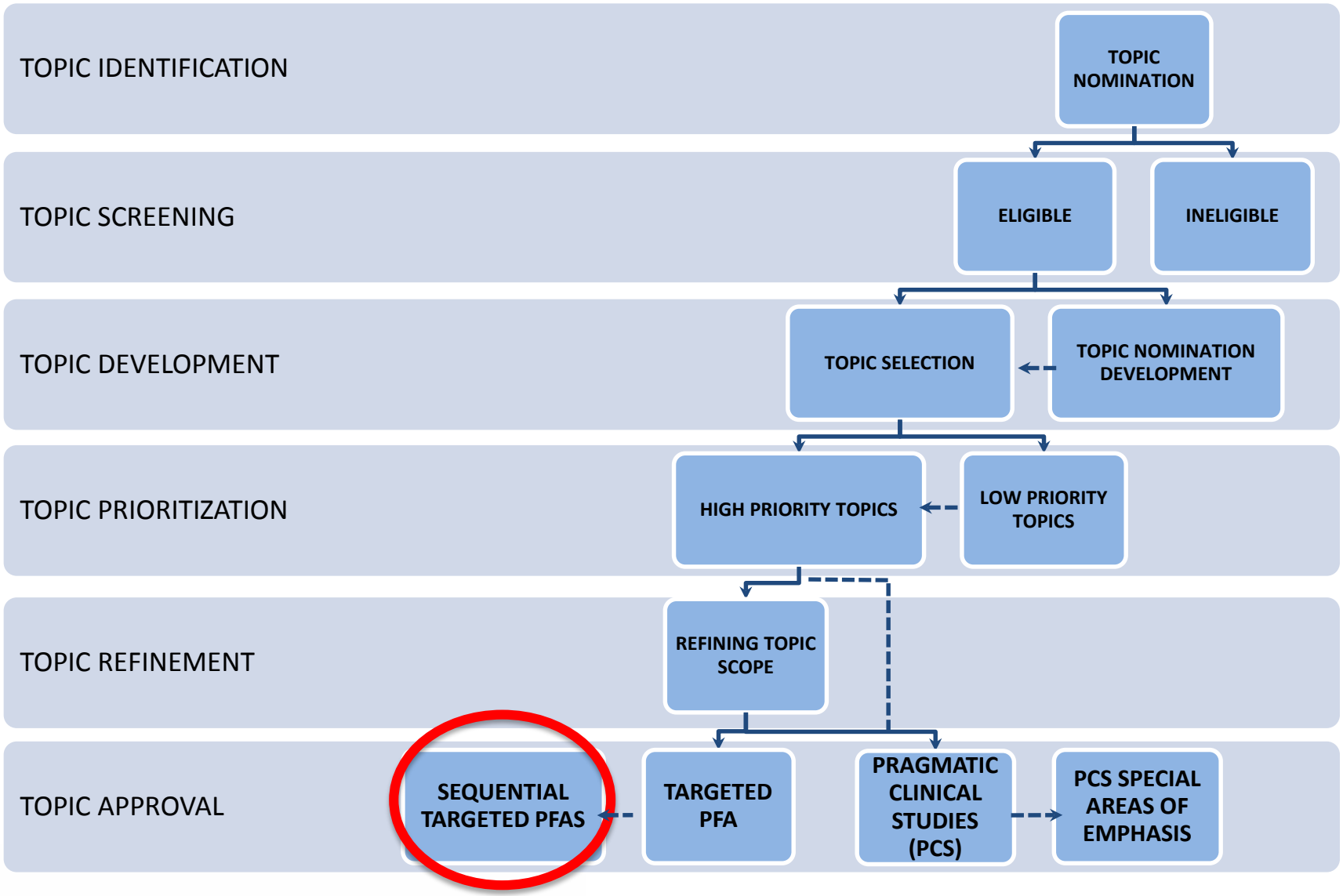
PCORI has released two opioid-specific targeted PFAs:

- Clinical Strategies for *Managing and Reducing Long-Term Opioid Use for Chronic Pain.* Cycle 3, 2015; re-issued Cycle 3 2016
- Strategies to *Prevent Unsafe Opioid Prescribing* in Primary Care among Patients with Acute or Chronic Non-Cancer Pain. Cycle 3 2016

Of 8 current PCORI-funded studies on opioids, two focus on treatment of opioid addiction, but neither focuses on pregnant women. One evaluates whether integrating opioid treatment into primary care settings is effective for low-income patients treated at federally qualified health centers, and the other evaluates whether extended release naltrexone improves outcomes for opioid-addicted prisoners re-entering society.

The proposed targeted PFA focuses on optimizing care for *pregnant and post-partum* women who use opioids.

PCORI Topic Prioritization Pathway



Medication-Assisted Treatment (MAT): Topic History

Topic Origin: Identified by Medicaid Medical Directors Network

Topic Trajectory:

- Discussed at multi-stakeholder workshop
- Discussed by Advisory Panel on Improving Healthcare Systems
- Approved by the SOC
- Recommended to the Board for approval

Stakeholder Input:

- Midwives, researchers, specialists, and Medicaid/medical directors

Rationale for This Targeted PFA

- **Prevalence of opioid use by pregnant women has increased dramatically**; associated with potentially serious maternal, fetal, and neonatal risks; evidence-based effective treatment is available
- **Medication-Assisted Treatment** (MAT; maintenance therapy with opiate agonist [methadone, buprenorphine] plus psychosocial services) improves outcomes: adherence to prenatal care, maternal weight gain, and neonatal birth weight; decreases opiate use and reduces criminal activity
- **Buprenorphine is safer than methadone**, reduces neonatal withdrawal issues, improves birth outcomes compared with methadone

Enhancing Opioid Treatment for Pregnant Women

- Pregnancy may motivate women to seek treatment, but there are treatment barriers: stigma (treatment setting and treatment type), lack of access, legal consequences
- Buprenorphine can be offered in physician's office (Drug Addiction Treatment Act), but requires qualification and delivery is not widespread
  - Fewer than half of counties in US have Office-Based Opioid Treatment (OBOT)
  - Percentage of qualified clinicians (2015):
    - Ob-gyn: 1%
    - Family Medicine: 22%
- Provider barriers include concerns re: lack of expertise, of adequate support, or mental health providers
- **Patient and provider barriers underscore the need to compare successful models of supporting opioid use disorder treatment for pregnant women**

Models of MAT Delivery for Pregnant Women

Existing models

- Vary by **integration**:
  - **Integrated** prenatal care, OBOT, addiction medicine, psychosocial service (NH, NM, OR)
  - **Co-located** prenatal care, OBOT, collaboration with community psychosocial services (WV)
  - Prenatal care by clinician, **referral** to opioid use disorder treatment **(usual care)**
- Offer **remote support** for providers:
  - Hub and spoke (NM)
    - Core components spokes: prenatal care and OBOT by clinician
    - More or less resource-intensive
      - Induction and stabilization (in methadone clinic or hospital vs office-based)
      - Psychosocial services (medical management vs referral; group vs individual)

Proposed Research Questions

1. What is the comparative effectiveness of alternative models for comprehensive opioid use disorder treatment delivery on maternal and neonatal outcomes in pregnant and post-partum women with different levels of addiction severity?
    - Comprehensive care includes prenatal care, medication-assisted treatment for addiction, psychosocial care

2. What is the comparative effectiveness of remotely supported opioid use disorder treatment delivery to pregnant women that includes more versus less resource-intense approaches to induction and psychosocial support for OBOT, in terms of maternal and neonatal outcomes?

Outcomes and Timing

- **Outcomes:**
  - **Addiction-specific:** Illicit drug use, relapse, treatment entry, treatment retention, patient quality of life, anxiety/depression
  - **Perinatal and Maternal:** Preterm birth, pregnancy complications, birthweight, neonatal complications, neonatal withdrawal issues
- **Duration:**
  - Repeated assessments to measure maternal and neonatal outcomes during pregnancy as well as 3 months post-partum

Population

- Pregnant women with opioid use disorder, infants born to women with opioid use disorder
  - Medicaid and private insurance
- Interested in heterogeneity of treatment effects among subgroups (e.g., addiction severity, low income or disadvantaged)

Interventions and Comparators

- Delivery models that may be compared include:
  - **Integrated** prenatal care, OBOT, and psychosocial services
  - **Co-located** prenatal care and OBOT, collaboration with community psychosocial services
  - Prenatal or addiction care and **referral** to addiction clinic or prenatal care **(usual care)**
  - Remote support for provider via **hub and spoke** approaches that vary by (1) induction and stabilization (clinician's office or addiction clinic or hospital) or (2) delivery of psychosocial services (medical management, co-located or integrated mental health provider, or referral)

Research Commitment

- The **total commitment** recommended for this targeted PFA is up to **$16 million** in total costs
  - Estimated number of studies: 3-4
  - Total direct costs: $3 million per study
  - Project period: 3-4 years

Timeline

Action	Date
SOC Endorsement	April 7, 2017
Board of Governors Vote	**May 8, 2017**
Preannouncement Released	May 9, 2017
Targeted PFA Announced	June 23, 2017
Letter of Intent Due	July 25, 2017
Application Deadline	October 25, 2017
Merit Review	January 9, 2018
Awards Announced	May 2018

Board Vote

Call for a Motion to:	• **Approve** $16M (total costs) for the development of a Targeted PFA for Medication-Assisted Treatment Delivery for Pregnant Women with Substance Use Disorders involving Prescription Opioids and/or Heroin
Call for the Motion to Be Seconded:	• **Second** If further discussion, may propose **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

Symptom Management for Patients with Advanced Illness

Recommendation for Targeted PFA Development Approval

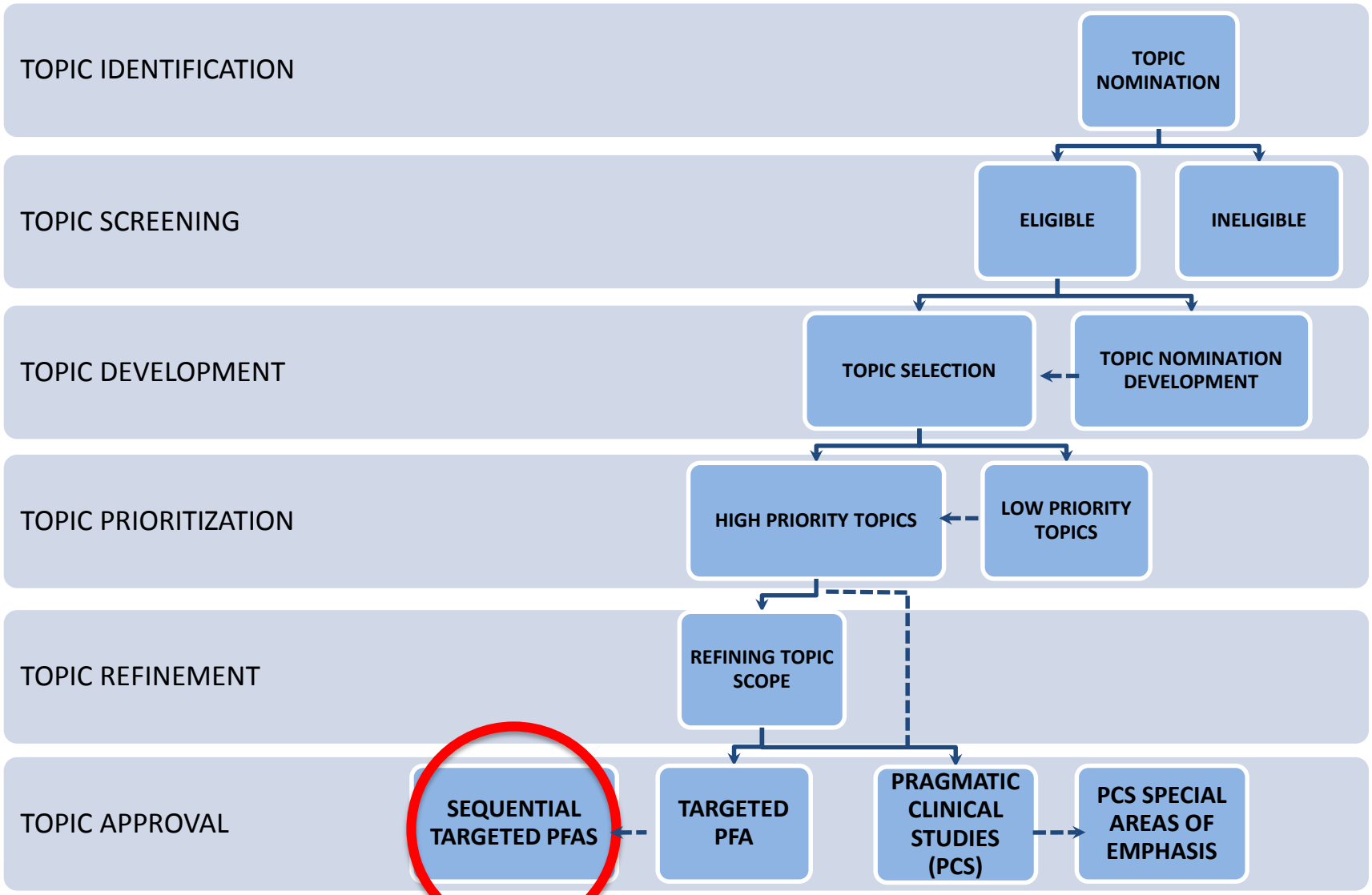
Robert Zwolak, MD, PhD

Chair, Science Oversight Committee

Evelyn P. Whitlock, MD, MPH

Chief Science Officer

PCORI Topic Prioritization Pathway



Recent Reports and PCORI Stakeholders Emphasize Need for Symptom Management Research in Advanced Illness

- PCORI's Palliative Care Delivery for Adult Patients with Advanced Illnesses and Their Caregivers: A Stakeholder Workshop (2016)
- **Cancer Moonshot Blue Ribbon Panel** (2016) **and James Lind Alliance** (2015) identified symptom management as a top research priority
- **IOM** (2014) **and NEJM review** (Kelley & Morrison, 2015) called for randomized controlled trials of different approaches to symptom management, especially in pediatric populations
- Discussions with pediatric palliative care experts from **AAP Section on Hospice and Palliative Medicine** underscore these evidence gaps (2016):
  - Need for research outside of cancer populations
  - Consideration of caregiver outcomes (e.g.. anxiety, depression)
- **Advisory Panel on Assessment, Prevention, Diagnosis, and Treatment Options** members expressed strong interest in the topic of symptom management for patients with advanced illness (2016)

Background

Palliative care improves patient well being in those with serious illness

- Systematic reviews show that patients with advanced illnesses who receive palliative care services report clinically meaningful improvements in quality of life, lower symptom burden, lower caregiver distress, and reduced hospitalizations (Dy et al., 2012; ICER, 2016; Gomez et al., 2013)

But significant gaps in research about how best to support patients and families persist

- Reports have identified three specific areas of research need:
  1. Models of care delivery
  2. Advanced care planning
  3. Symptom management
     - *Center to Advance Palliative Care, 2015; IOM, 2014; Kelley & Morrison, 2015*

PCORI's Focused Funding to Date

1. **Models of care delivery** ✓
   - *Comparative effectiveness of established models of palliative care in adults in community settings – impact on patient-reported outcomes and caregivers*
   - Targeted PFA released in August 2016

2. **Advanced care planning** ✓
   - *Comparative effectiveness of approaches to facilitating advance care planning conversations in adults – impact on patient-reported outcomes and caregivers*
   - Targeted PFA released in August 2016

3. **Symptom management**
   - *Comparative effectiveness of treatment options for relief of common symptoms across multiple advanced illnesses (e.g. pain, fatigue, difficulty breathing, insomnia)*
   - Planned sequential targeted PFA – If approved, released June 2017 for potential awards in May 2018

Rationale for Sequential Targeted PFA on Symptom Management

- Remaining top priority for research in this area
  - Where studied, symptom management for patients with advanced illness **does not reflect the range of diseases and patient populations affected**
  - Multiple treatments for each symptom with evidence of efficacy and/or widespread use, but **few head-to-head trials**
  - Existing studies generally have **small sample sizes**

Proposed Research Question

What is the comparative effectiveness of two or more interventions, including at least one pharmacologic intervention, for symptom management in patients with serious advanced illness?

- **Symptoms may include:** pain, fatigue, dyspnea, insomnia, anorexia-cachexia, nausea/vomiting, and depression/anxiety
- Studies should examine one or more symptoms

Population

Population: Patients with **advanced, life-limiting illness** and their caregivers

- Conditions may include but *are not limited to*:
  - Advanced heart failure
  - Advanced cancers
  - COPD
  - End-stage liver or kidney disease
  - Advanced neurodegenerative diseases
- PCORI has a special interest in **pediatric patients and their caregivers**

Interventions and Comparators

Intervention(s): At least one pharmacologic intervention

Comparator(s): Other pharmacologic or non-pharmacologic comparators

- Proposed interventions and comparators must:
  - Have at least moderate evidence of efficacy and/or be in widespread use and be capable of delivery in a standardized format
  - Address actual clinical choices faced by patients, their caregivers, and clinicians in specific practice settings

Outcomes and Timing

Outcomes:

- Patient-centered outcomes such as quality of life (e.g., MQOL, QUAL-E)
- Caregiver outcomes (e.g., burden)
- Symptom outcomes
- Adverse effects, including unintended effects of symptom treatment on other symptoms and patient and/or caregiver experience (e.g., delirium, which may be made worse by some treatments)

Duration: At least 6 months of follow up

Research Commitment

- The **total commitment** recommended for this targeted PFA is up to **$25 million** in total costs
  - Estimated number of studies: 8-10
  - Total direct costs: $2 million per study*
  - Maximum project period: 3 years*

*For studies of uncommon conditions, PCORI would consider funding larger and/or longer studies if investigators provide a strong rationale.

Timeline

Action	Date
SOC Endorsement	April 7, 2017
Board of Governors Vote	**May 8, 2017**
Preannouncement Released	May 9, 2017
Targeted PFA Announced	June 23, 2017
Letter of Intent Due	July 25, 2017
Application Deadline	October 25, 2017
Merit Review	January 2018
Awards Announced	May 2018

Board Vote

Call for a Motion to:	• **Approve** $25M (total costs) for the development of a targeted PFA for Symptom Management for Patients with Advanced Illness
Call for the Motion to Be Seconded:	• **Second** 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

FY2017 Mid-Year Financial Review (As of 3/31/2017)

Larry Becker
Chair, Finance and Administration Committee
Regina Yan, MA
Chief Operating Officer

FY2017 Budget vs. Actual by Broad Categories
(As of 3/31/2017)

	Annual Budget FY2017	Budget thru 3/31/17	Actual thru 3/31/17	Variance thru 3/31/17 ($)	%
PROGRAM EXPENSE	$ 379,153,554	$ 173,969,712	$ 163,441,340	$ 10,528,372	6%
PROGRAM SUPPORT	14,515,211	7,558,888	7,946,851	(387,963)	-5%
ADMINISTRATIVE SUPPORT	29,407,689	14,600,419	14,679,916	(79,497)	-1%
TOTAL	$423,076,454	$196,129,019	$186,068,107	$ 10,060,912	5%

FY2017 Budget vs. Actual Percentages
(As of 3/31/2017)

	FY2017 YTD BUDGET (through 3/31/17)	% of Total Budget	FY2017 YTD ACTUAL (through 3/31/17)	% of Total Actual
PROGRAM EXPENSE	$ 173,969,712	89%	$ 163,441,340	88%
PROGRAM SUPPORT	7,558,888	4%	7,946,851	4%
ADMINISTRATIVE SUPPORT	14,600,419	7%	14,679,916	8%
TOTAL	$ 196,129,019	100%	$ 186,068,107	100%

Wrap Up and Adjournment

Grayson Norquist, MD, MSPH
Chairperson, Board of Directors