Board of Governors Meeting
via Teleconference/Webinar

May 23, 2016

10:00 a.m. -5:45 p.m. 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

Welcome, Call to Order and Roll Call
Q2 Dashboard Review
Mid-Year Financial Review
Methodology Committee (MC) Update
Consider for Approval: PCORnet Cross-PPRN Research Project Award
Report on Application Enhancement Efforts
Stakeholder Panel: Specialty Physician Organizations
Dissemination Strategy
Consider for Approval: Targeted PCORI Funded Announcements • Management of Sickle Cell Disease • Strategies to Prevent Unsafe Opioid Prescribing in Primary Care • Community-based Palliative Care Delivery for Adult Patients
Consider for Approval: Additional Awards from Previous Cycles
Portfolio Analysis – Depression, Pain, Sleep, and Fatigue Outcomes

Consent Agenda Items

Grayson Norquist, MD, MSPH
Chairperson, Board of Governors

Motion for Consent Agenda Items

That the Board approve:

- Minutes from April 26, 2016 Board meeting
- Nomination of Dr. Andrew Bindman to serve on the Engagement, Dissemination and Implementation Committee (EDIC) and the Science Oversight Committee (SOC)
- The Updated Research and Infrastructure Project Budget Increase Policy

Board Vote

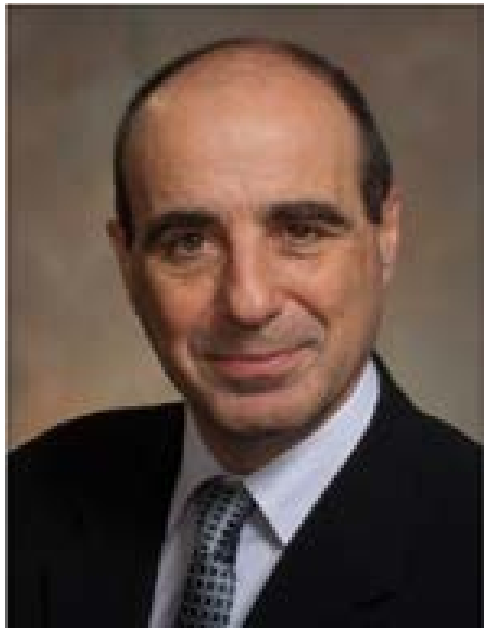
Call for a Motion to:	• **Approve** each of the Motions on the Consent Agenda
Call for the Motion to Be Seconded:	• **Second** the Motion • If further discussion, may propose an **Amendment** to the Motion or an **Alternative** Motion
Voice Vote:	• Vote to **Approve** the **Final** Motion • Ask for votes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

Executive Director's Report

Joe Selby, MD, MPH
Executive Director

Welcome, Dr. Andrew Bindman!

- Andrew Bindman, MD, is the newest member of the PCORI Board of Governors
- Recently named Director of the Agency for Healthcare Research and Quality (AHRQ)
- His career as a primary care physician and health services researcher shows great commitment to the care of vulnerable populations and the translation of research into policy
- Dr. Bindman has served as Professor of Medicine, Epidemiology and Biostatistics at the University of California, San Francisco; Robert Wood Johnson Health Policy Fellow; and Senior Advisor to the Centers for Medicare & Medicaid Services, among others. He was elected to the National Academy of Medicine in 2015



BMJ: Partnering with Patients

- In June 2014, the British Medical Journal launched an initiative to incorporate the patient perspective
  - The level of patient involvement is now a stated field in all research papers
  - Patients have also been incorporated into the peer review process and the BMJ editorial board
- BMJ cited PCORI as an organization sharing this patient-centered mission

> "Discussion groups of patients, carers, and clinicians led by … the Patient Centered Outcomes Research Institute in the United States, are *shedding light on the mismatch between the questions that patients and doctors want answers to and the ones that researchers are investigating*."

'Let the patient revolution begin'
BMJ 2015

Stakeholder Workshop: Hepatitis C

- A Stakeholder Workshop on Hepatitis C took place on May 18th as a follow-up to the October 2014 meeting on the topic
- Participation was invite-only and the public was able to virtually attend
- 33 stakeholders participated, in addition to six PCORI Board members and 17 public audience members

- **Next Steps:**
  - The Board of Governors will discuss the feedback from this workshop and determine whether to pursue a targeted PFA

2016 Annual Meeting – Overview and Goals

- Nov. 17-19, Gaylord National Harbor Hotel
- ~1,000 members of PCORI community; “Patients Included”
- Plenaries/breakouts, summits, networking opportunities; guided by stakeholder steering committee/staff program committee
- Goals
  - Spotlight PCORI’s leadership role in CER/PCOR
  - Focus on emerging results of funded studies as well as projects in progress Convene awardees/stakeholders to advance discussion of topics of interest, provide new partnership opportunities, inform future work
  - Highlight our emerging dissemination work and efforts to advance knowledge sharing

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Dashboard Review
Second Quarter of FY-2016

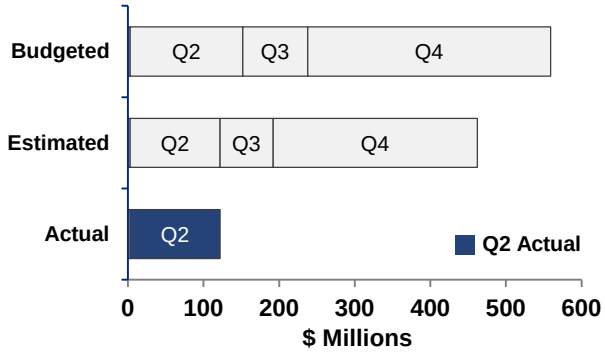
Joe Selby, MD, MPH
Executive Director

Board of Governors FY-2016 Dashboard
Q2 (As of 3/31/2016)
Our Goals: Increase Information, Speed Implementation, and Influence Research

On Target | Off Target | Needs Attention | Projected/Target | Q3 2015 | Q4 2015 | Q1 2016 | Q2 2016

Inputs → Process → Outputs → Uptake → Use → Impact

Funds Committed to Research, Up to $554M



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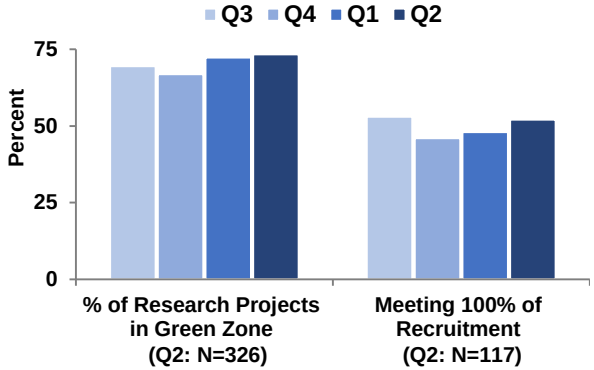
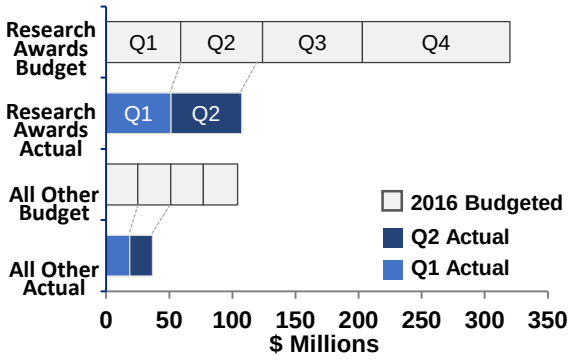


Expenditures – Total Budget, Up to $424M



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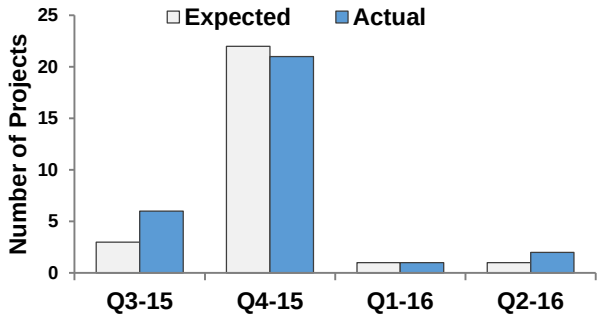

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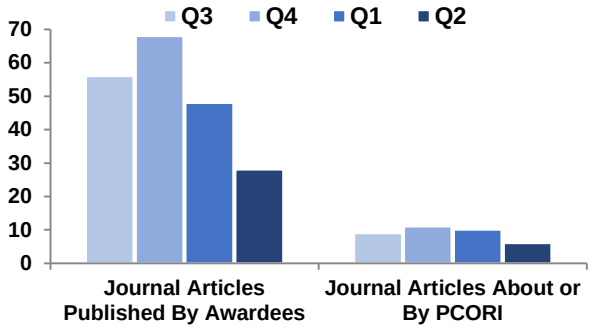
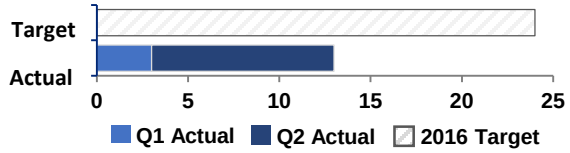
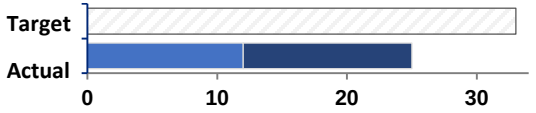


PCORnet Phase II

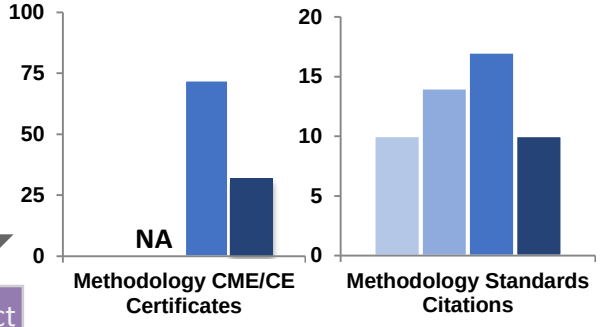
Research Projects Underway in PCORnet



Networks Engaged in Research Projects



Methodology Standards Uptake



Highlight: Specialty Physicians & PCOR

Two clinical specialties, Nephrology and Radiology, are working in their fields to implement PCORI Methodology Standards, drive a useful portfolio, and promote uptake of PCORI research results

Results: Influencing Research

Catalyzed by PCORI, the Meharry-Vanderbilt Alliance developed pre- and post-doctoral programs in community engaged research, and began including stakeholders in their grant review process

Results of Engagement in Research

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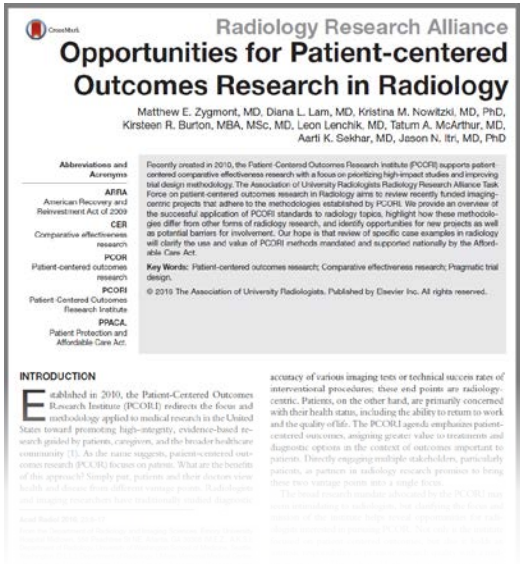
Specialty Physicians Engaging with PCORI to drive a useful portfolio and facilitate uptake of results

Radiology

The Association of University Radiologists Radiology Research Alliance Task Force reviewed all PCORI-funded radiology projects, and described how the PCORI Methodology Standards apply to medical imaging. **The Task Force identified opportunities for future projects in the field, and developed a National Agenda for PCOR.**

> *As radiologists, we must embrace PCOR or risk missing a key opportunity to demonstrate value and improve the care provided to our patients.*


Radiology Research Alliance

Opportunities for Patient-centered Outcomes Research in Radiology

Matthew E. Zygmont, MD, Diana L. Lam, MD, Kristina M. Nowitzki, MD, PhD, Kirsteen R. Burton, MBA, MSc, MD, Leon Lenchik, MD, Tatum A. McArthur, MD, Aarti K. Sekhar, MD, Jason N. Itri, MD, PhD

Abbreviations and Acronyms
ARRA American Recovery and Reinvestment Act of 2009
CER Comparative effectiveness research
PCOR Patient-centered outcomes research
PCORI Patient-Centered Outcomes Research Institute
PPACA Patient Protection and Affordable Care Act.

Recently created in 2010, the Patient-Centered Outcomes Research Institute (PCORI) supports patient-centered comparative effectiveness research with a focus on prioritizing high-impact studies and improving trial design methodology. The Association of University Radiologists Radiology Research Alliance Task Force on patient-centered outcomes research in Radiology aims to review recently funded imaging-centric projects that adhere to the methodologies established by PCORI. We provide an overview of the successful application of PCORI standards to radiology topics, highlight how these methodologies differ from other forms of radiology research, and identify opportunities for new projects as well as potential barriers for involvement. Our hope is that review of specific case examples in radiology will clarify the use and value of PCORI methods mandated and supported nationally by the Affordable Care Act.

Key Words: Patient-centered outcomes research; Comparative effectiveness research; Pragmatic trial design.

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INTRODUCTION

Established in 2010, the Patient-Centered Outcomes Research Institute (PCORI) redirects the focus and methodology applied to medical research in the United States toward promoting high-integrity, evidence-based research guided by patients, caregivers, and the broader healthcare ...

... accuracy of various imaging tests or technical success rates of interventional procedures; these end points are radiology-centric. Patients, on the other hand, are primarily concerned with their health status, including the ability to return to work and the quality of life. The PCORI agenda emphasizes patient-centered outcomes, assigning greater value to treatments and ...

Zygmont ME, et al. **Opportunities for Patient-Centered Outcomes Research in Radiology**. *Acad Radiology*. Jan 2016; 23:8-17.

Nephrology

In an interview on becoming Editor in Chief of the American Journal of Kidney Diseases, Harold Feldman, MD, MSCE, Director of the Center for Clinical Epidemiology and Biostatistics at the University of Pennsylvania, highlighted the major studies related to Nephrology that PCORI is funding, and said **the journal will focus on translating findings from PCOR into clinical practice.**

Goal 3 Results: Influence Research

Meharry-Vanderbilt Alliance

Consuelo H. Wilkins, MD

PCORI is credited with being a catalyst for:

- **Inclusion of community members and stakeholders in Scientific Review Process for the Vanderbilt Clinical and Translational Science Award Pilot Program**
  - Observing PCORI's review process encouraged them to think more broadly about inclusion of stakeholders in the scientific review and their ability to contribute to proposal review
  - Developing training curriculum based on PCORI's Mentor Program
- **Post-Doctoral Research Fellow Program in Community Engaged Research**
  - 2-year program for training researchers in community engagement
  - 2 full-time fellows are supported each year by the NIH CTSA
- **Community Scholars Program for pre-doctoral students**
  - 1-year immersion experience in community engaged research
  - Includes research partnership and mentoring
  - $5,000 to support the research project and $500 stipend to the community organization

> *The motivation of researchers because of PCORI funding has been a big stimulus for the work that we do… the availability of PCORI funding and the interest was a catalyst for us to be able to expand our reach.*
>
> Consuelo Wilkins, MD

Results of Engagement in Research: Stakeholder Involvement Led to Improved Enrollment

Minneci PC, Nacion KM, Lodwick DL, Cooper JN, Deans KJ. **Improving Surgical Research by Involving Stakeholders**. *JAMA Surgery*. Feb 2016.

- Awarded 2013, Assessment of Prevention, Diagnosis, and Treatment Options project
- Principal Investigator: Katherine Deans, MD, Nationwide Children's Hospital

In this PCORI-funded study of a patient activation tool (part of a larger comparison of surgery vs. antibiotics to treat pediatric appendicitis), **stakeholders provided suggestions help improve enrollment and retention rates,** including making the enrollment script more patient- and family-centered and offering an online option for follow-up.

These changes **increased enrollment in the trial from 65% to 95% and increased retention from 58% to 85%.**

In the **Top 5%** of research outputs scored by Altmetric

> *These are tangible statistics that show that this process of involving the stakeholders can improve the study.*
>
> Dr. Minneci, Co-Investigator

> *This is why we have our stakeholder group, so that we can incorporate their input into all phases of the study. In this situation, it was critical to the success of our project.*
>
> Dr. Deans, Principal Investigator

Nationwide Children's Hospital. "Stakeholder involvement in clinical trial design leads to greater recruitment, retention." ScienceDaily, 10 February 2016.

Progress of Pragmatic Clinical Studies

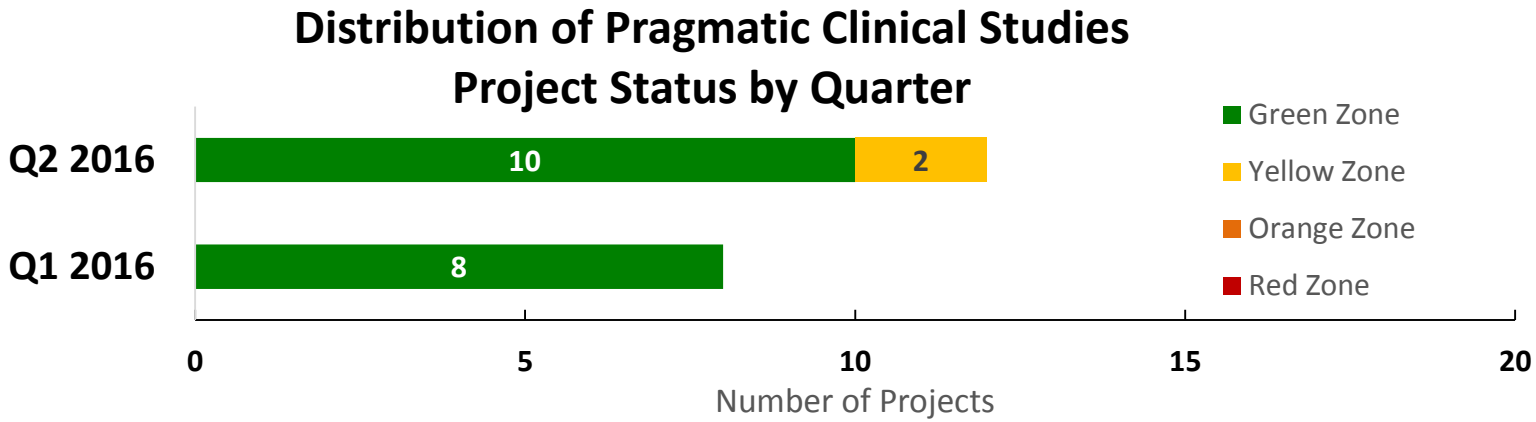
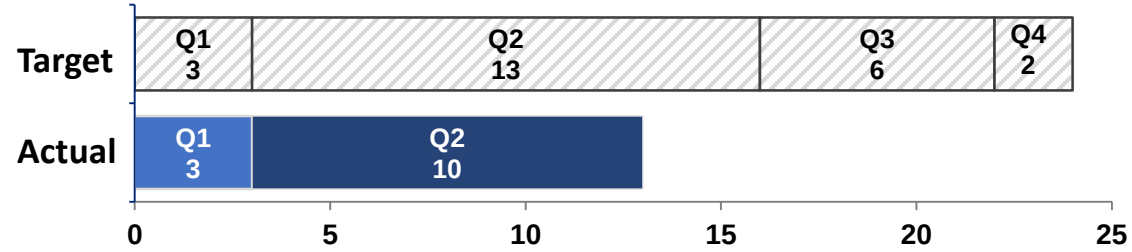


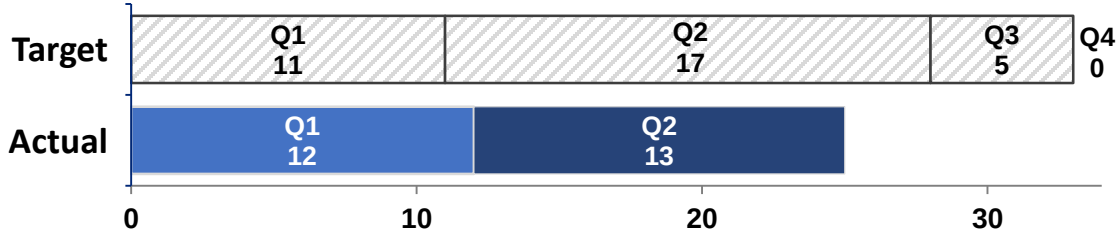
Table 1. Active Research Project Details	Q3-15	Q4-15	Q1-16	Q2-16
Number of Active Research Projects (Contracts Executed, have passed the Start Date)	0	7	10	14
Number of Projects Eligible for First Evaluation (Active projects far enough along to be categorized based on progress)	**-**	**0**	**8**	**12**
Percent of Projects on Track (in the Green Zone)	-	-	100%	83%
Number of Projects with Recruitment Milestones in Quarter	-	-	**0**	**4**
Percent of Projects Meeting **100%** of Recruitment Milestones	-	-	-	50%

Progress of PCORnet Phase II

Research Projects Under Way in PCORnet

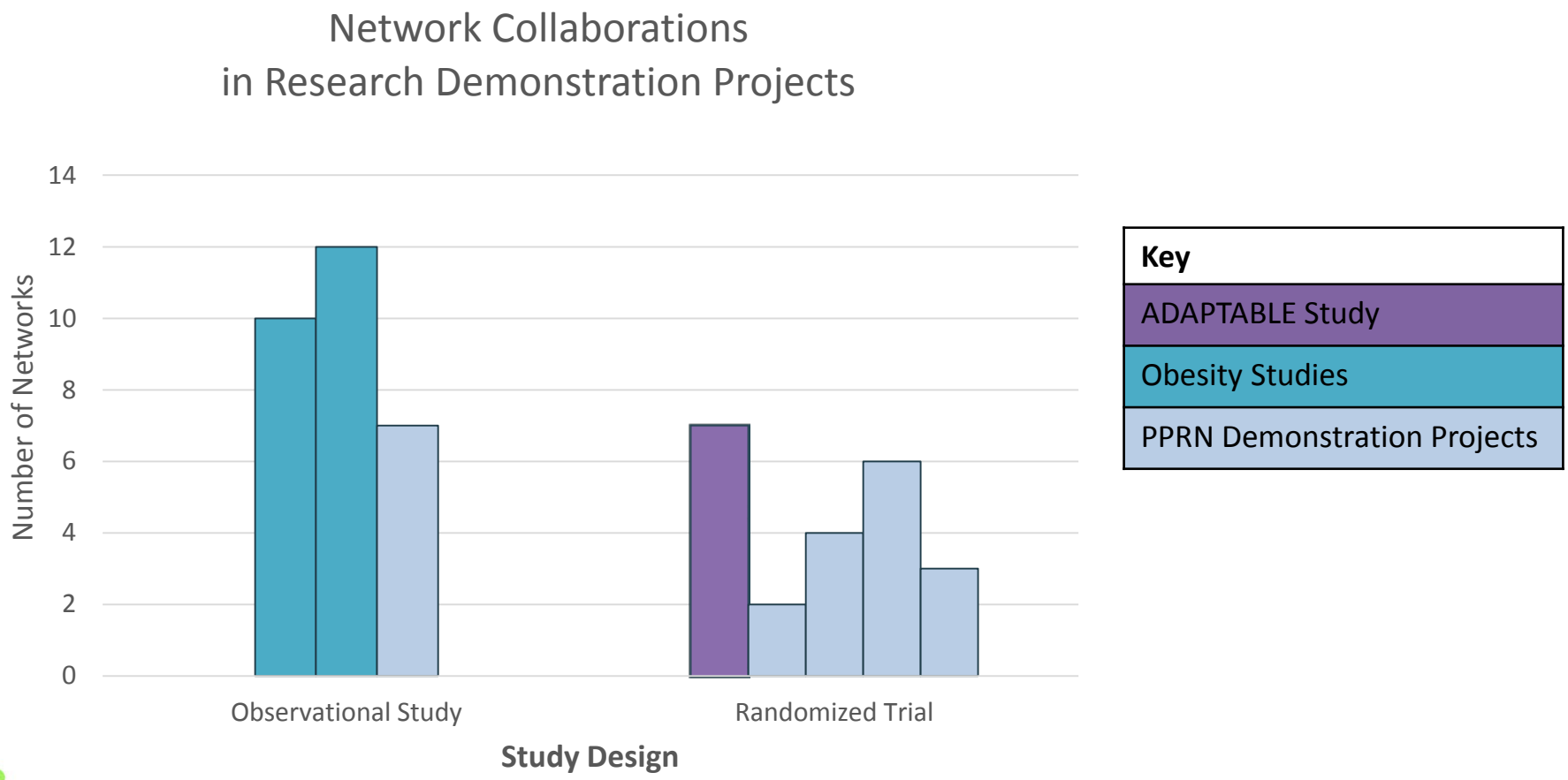


Networks Engaged in Research Projects



PCORnet Network Collaboration

There are currently 8 collaborative Research Demonstration projects taking place in PCORnet involving 25 of 33 networks.



PCORnet Front Door Policy

April 5, 2016 — **Internal Launch of PCORnet Front Door**

April 15, 2016 — **Front Door Policy Approved**

Summer 2016 — **Public Opening of PCORnet Front Door**

Inquiry Types
(as of April 15, 2016)

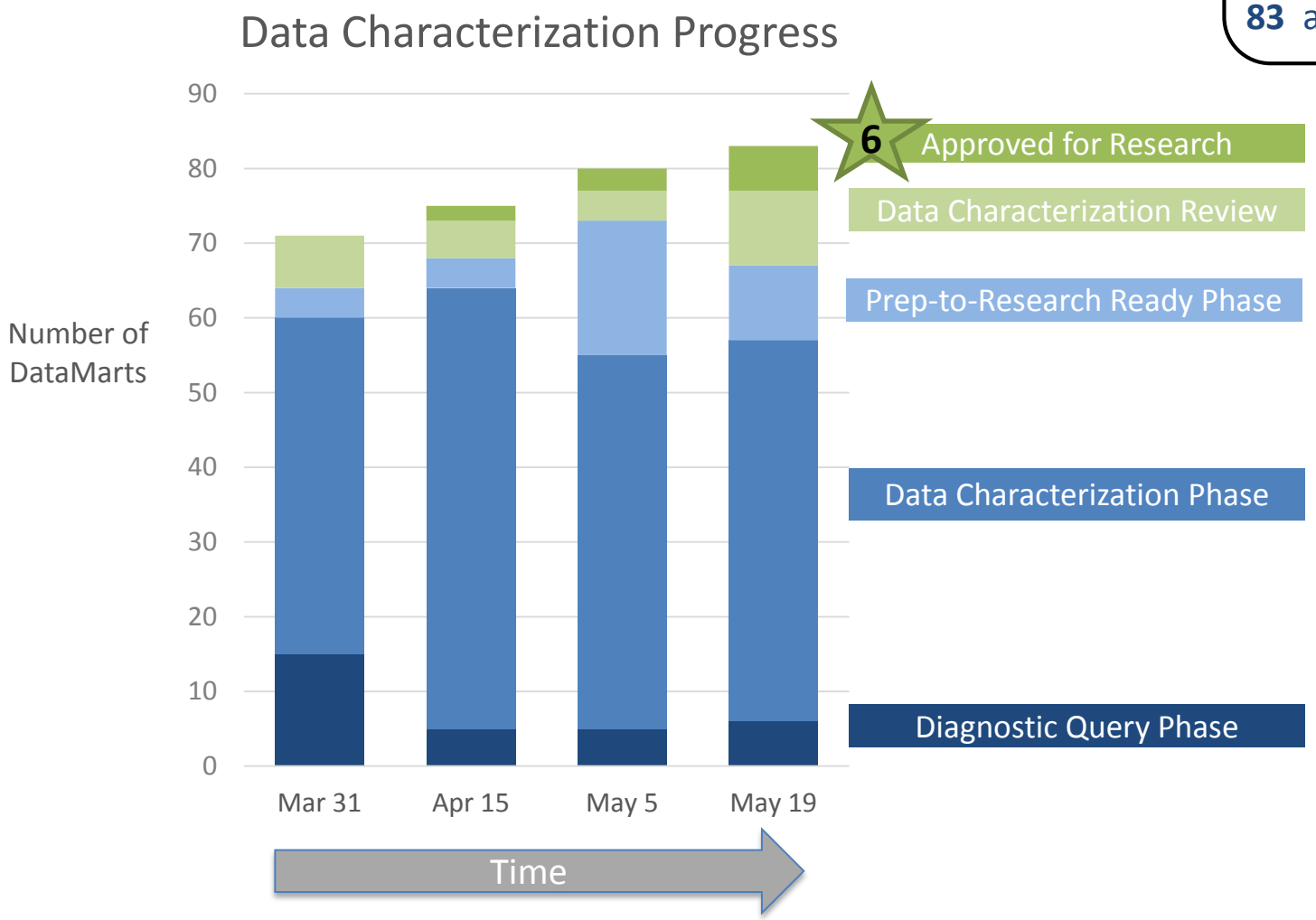
- *13 Trials*
- *4 Observational Studies*
- *2 Participating Sites Inquiries*

Requester Types
(as of April 15, 2016)

- *8 Academic*
- *6 Industry*
- *2 Non-profit/Foundation*
- *1 National Association*
- *1 Federal*
- *1 Research Center*

Research-Ready PCORnet

DataMart Totals:
71 as of March 31st
75 as of April 15th
80 as of May 5th
83 as of May 19th



Discussion Questions for Q2-16:

- Do our FY-2016 Dashboard and associated background materials cover the ***topics that are most important for your review***?
- Do you have ***questions or comments about our progress or performance*** on any of our Dashboard indicators?
- Does the in-depth focus this quarter on ***the progress of PCORnet*** tell you what you need to know?

Board of Governors FY-2016 Dashboard
Q2 (As of 3/31/2016)
Our Goals: Increase Information, Speed Implementation, and Influence Research

On Target | Off Target | Needs Attention | Projected/Target | Q3 2015 | Q4 2015 | Q1 2016 | Q2 2016

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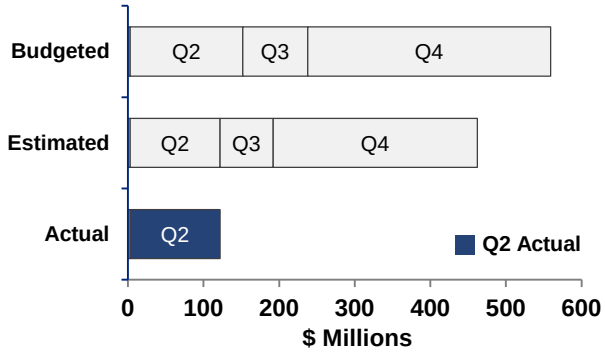

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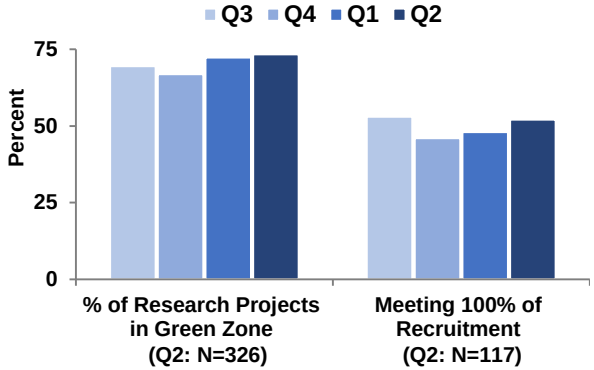

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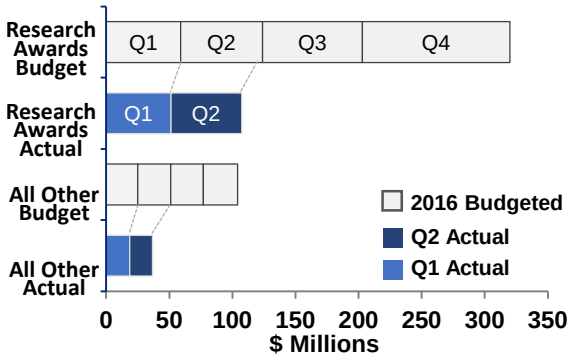

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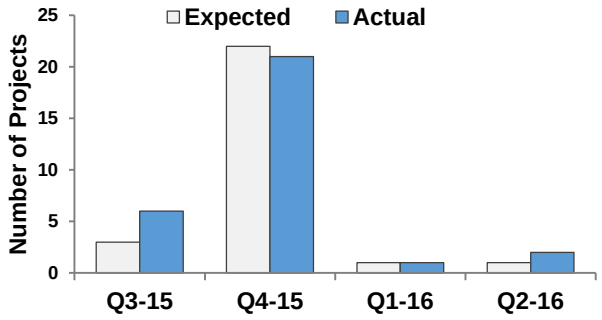

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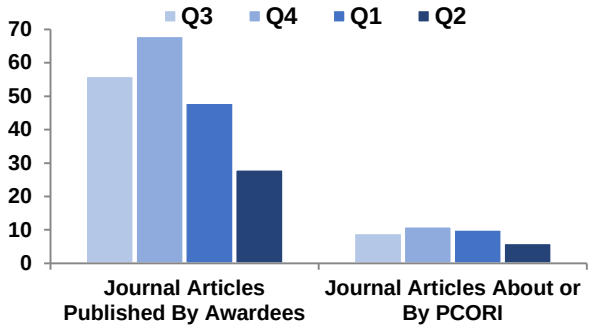
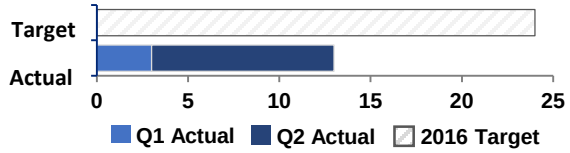


PCORnet Phase II

Research Projects Underway in PCORnet



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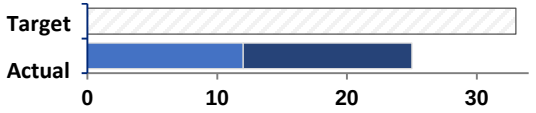

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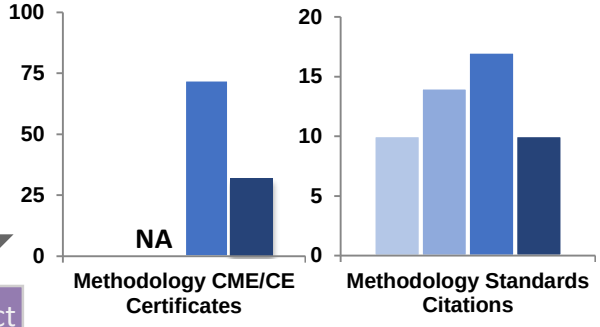

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FY2016 Mid-Year Financial Review (As of 3/31/2016)

Larry Becker
Chair, Finance and Administration Committee

Regina Yan, MA
Chief Operating Officer

Overview

- Summary
  - PCORI Revenue and Cash Balance
  - Research and Other Programmatic Funding Commitments
- Budget vs. Actuals Review (as of 3/31/2016)
  - FY2016 Budget vs. Actual by Broad Categories
  - FY2016 Budget vs. Actual Percentages
  - Top Three Factors in Variance
- Funding Commitment Plan: FY2012 - FY2019
- PCORI Estimated Revenue and Expenditures

Summary: PCORI Revenue and Cash Balance

Cash Balance at 9/30/2015 ***($ in millions)***	**$ 816.5**
Revenue from 10/1/2015 - 3/31/2016	**214.4**
Federal Appropriation	*120.0*
CMS Transfers	*98.7*
PCOR Fee	*(5.0)*
Interest Income	*0.7*
Cash Disbursements	**(159.4)**
Cash Balance at 3/31/2016 in PCOR Trust Fund and bank account	**$ 871.5**

Note: As of March 31, 2016, there were outstanding award obligations of $936 million that will become due and payable as research progresses over time.

Summary: Research and Other Programmatic Funding Commitments

Cumulative Funding Commitments* (as of 3/31/2016)	**$1,327 million**
Outstanding Award Obligations (as of 3/31/2016)**	**$936 million**

* Includes Research, Infrastructure, and Engagement funding commitments.

** Outstanding award obligations are amounts of contracts awarded that will require payments during a future period. These amounts will become due and payable as research progresses over time.

FY2016 Budget vs. Actual by Broad Categories
(As of 3/31/2016)

	Annual Budget FY2016	Budget thru 3/31/16	Actual thru 3/31/16	Variance thru 3/31/16 ($)	%
Award Expense					
Research, Infrastructure, and Engagement Awards	$ 331,526,300	$ 129,769,402	$ 111,817,452	$ 17,939,074	14%
Program Support					
Methodology Committee	1,636,000	818,000	448,104	369,896	
Science	30,110,200	14,664,880	8,740,785	5,924,095	
Evaluation & Analysis	75,000	37,500	43,163	(5,663)	
Research Infrastructure	2,407,450	1,246,948	1,522,763	(275,815)	
Engagement & Dissemination	12,148,203	5,155,604	3,583,659	1,571,945	
Contracts Management & Administration	6,674,025	3,217,004	2,173,939	1,043,065	
Total Program Support	**53,050,878**	**25,139,936**	**16,512,413**	**8,627,523**	**34%**
Administrative Support					
Board of Governors	1,085,000	521,667	551,897	(30,230)	
Management and General	37,819,122	19,011,948	14,786,710	4,225,238	
Total Administrative Support	**38,904,122**	**19,533,615**	**15,338,607**	**4,195,008**	**21%**
TOTAL	**$423,481,300**	**$174,442,953**	**$143,681,349**	**$ 30,761,064**	**18%**

The variance for the same period in FY2015 was $41.4 million or 28%.

FY2016 Budget vs. Actual Percentages
(As of 3/31/2016)

	2016 Budget	% of Total Budget	Actual thru 3/31/16	% of Total Actual
Award Expense	$ 331,526,300	78%	$ 111,830,328	78%
Program Support	53,050,878	13%	16,512,413	11%
Administrative Support	38,904,122	9%	15,338,607	11%
TOTAL	$ 423,481,300	100%	$ 143,681,349	100%

Budget vs. Actual Review: Top Three Factors in Variance

Key Factors in Variance	Amount ($)	% of Total Variance
Award Expense	$17.9 million	58%
Salaries and Benefits	$3.4 million	11%
Evidence to Action Networks	$1.7 million	6%

Funding Commitment Plan: FY2012 – FY2019

FUNDING COMMITMENT PLAN ($ in millions)				
FISCAL PERIOD	**RESEARCH***	**INFRASTRUCTURE (PCORnet)****	**ENGAGEMENT**	**TOTAL**
Inception to FY2013	$ 272	$ -	$ -	$ 272
FY2014	305	103	3	411
FY2015	370	149	16	535
FY2016	415	43	24	482
FY2017	345	-	28	373
FY2018	345	-	27	372
FY2019	100	-	23	123
	$ 2,152	**$ 295**	**$ 120**	**$ 2,567**
	84%	11%	5%	100%

* Research funding commitments include $60 million in projects conducted within PCORnet.

** Infrastructure (PCORnet) funding commitments are CER capacity building investments that make the data and partnerships with patients, clinicians and researchers available to CER researchers, but does not actually invest in Research that uses this infrastructure, as does the Research funding. Infrastructure awards include funding for CDRN and PPRN networks, PCORnet coordinating centers, health plan infrastructure, and CMS linkage project to supplement CDRN data with Medicare claims.

PCORI Estimated Revenue and Expenditures

	In Millions	% of Total Expenditures
Revenue (thru FY2019)	$3,258	
Awards (Research/Infrastructure/Engagement)	$2,567	79%
Dissemination	$103	3% (or 4% of Awards)
Program Support	$310	10%
General Admin	$278	9%
Total Expenditures*	**$3,258**	**100%**

* $2.6 billion will be committed by FY2019. Expenses will continue through FY2024 until all research projects are completed.

Dissemination: Includes major PCORI dissemination activities, as well as funds provided to awardees to conduct dissemination

Program Support: Includes costs related to Methodology Committee, Science, Engagement, and Contract Management

General Admin: Includes costs related to the Board, administrative staff, rent, IT system infrastructure, etc

Methodology Committee Update

Robin Newhouse, PhD, RN

Chair, PCORI Methodology Committee

Methodology Committee Members

- Robin Newhouse, Chair
- Steven Goodman, Vice Chair
- Naomi Aronson
- Ethan Basch
- Stephanie Chang
- David Flum
- Cynthia Girman
- Mark Helfand
- Michael Lauer
- David Meltzer
- Brian Mittman
- Sally Morton
- Neil Powe
- Mary Tinetti
- Adam Wilcox

New MC member:
Stephanie Chang

Session Topics and Objectives

- Implementation of the PCORI Methodology Standards
- Update on the public comment period for the draft revisions to the PCORI Methodology Standards
- Coordinating with the Clinical Trials Advisory Panel
- Other updates
  - Network Research Methods work group
  - MC advisors

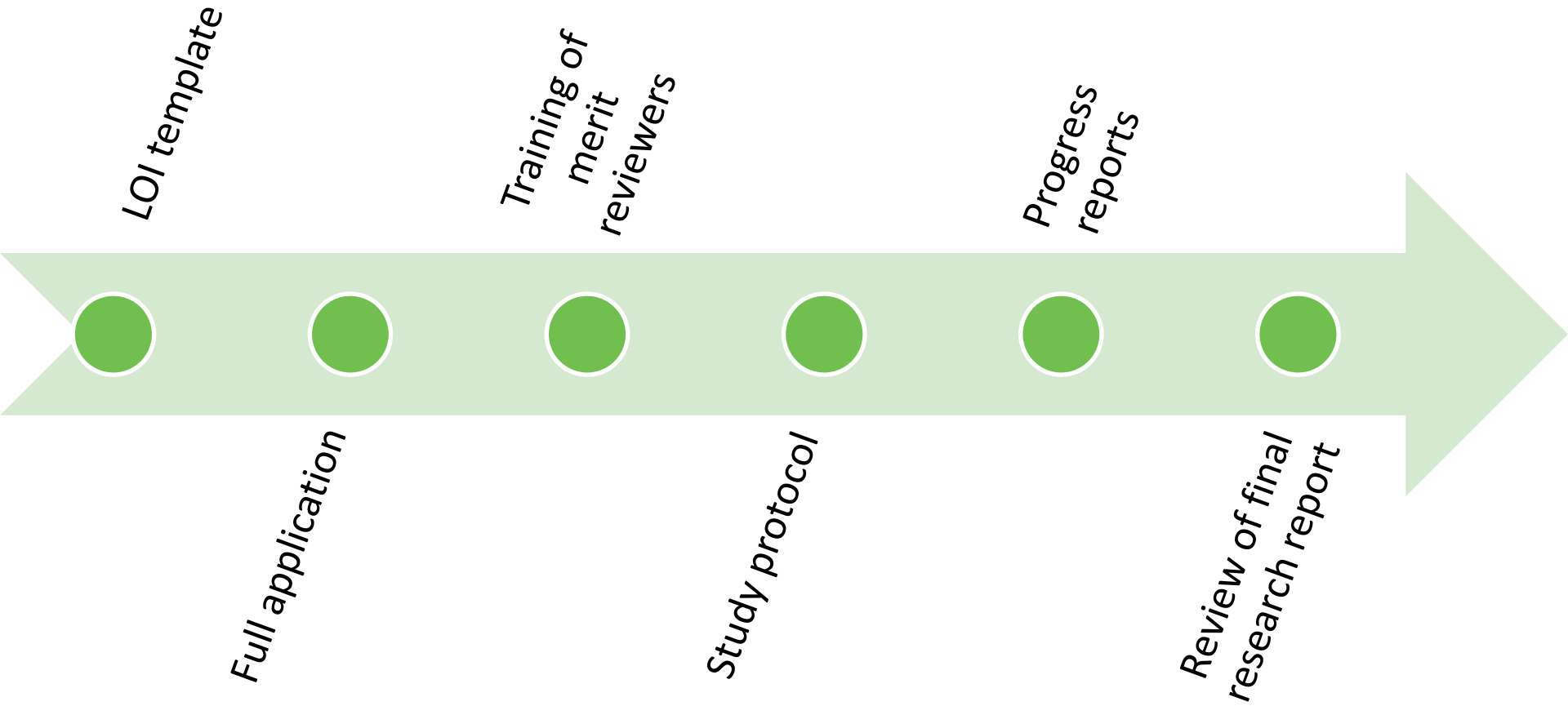
Goals of Implementation of the PCORI Methodology Standards

- Help investigators understand and use the Standards
- Establish a system for using the Standards to ensure methodological integrity of research projects funded by PCORI
- Identify barriers to use of the Standards

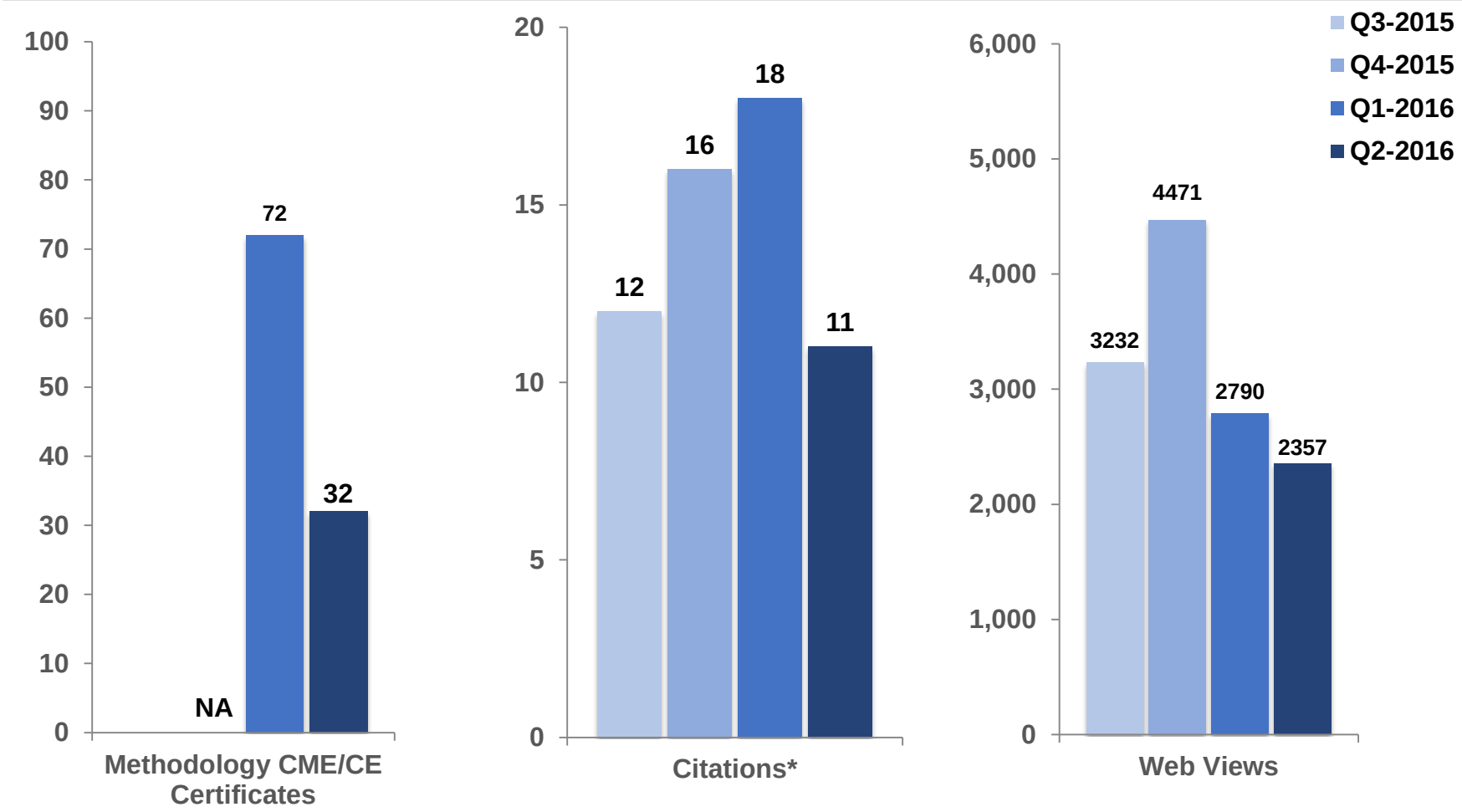
Helping Researchers Understand and Use the Standards

- Webinars for applicants (launched in 2013)
- PCORI outreach conferences (launched in 2014)
- Online CME program (launched in 2015)
- Academic curriculum (launched in 2016)

Using the Standards to Ensure Methodological Integrity of Funded Projects



Uptake of Methodology Standards



*PCORI Methodology Committee. **Methodological standards and patient-centeredness in comparative effectiveness research: the PCORI perspective**. *JAMA*. 2012 Apr 18;307(15):1636-40.

Public Comments on the Draft Revisions to the Methodology Standards

- Public comment period open between January and April, 2016
- Total comments: 84
- Stakeholder groups represented:
  - Health researchers
  - Industry
  - Caregivers/family members
  - Patients
- The public comments will guide final revisions to the Methodology Standards and will be summarized in the revised PCORI Methodology Report

Overview of Public Comments

Standard Category	Number of comments
Formulating Research Questions	22
Patient-Centeredness	12
Data Integrity and Rigorous Analysis	9
Preventing and Handling Missing Data	4
Heterogeneity of Treatment Effects	5
Data Registries	3
Data Networks as Research-Facilitating Structures	5
Causal Inference Methods	12
Adaptive Trial Designs	3
Studies of Diagnostic Tests	4
Systematic Reviews	0
Research Designs Using Clusters	5

PCORI's Clinical Trials Advisory Panel

- Established to advise PCORI and other entities on best practices for clinical trials
- Close collaboration with and oversight by the Methodology Committee
- Types of advice and resources
  - Strategies for development of the PCORI clinical trials portfolio
  - Guidance on the conduct of clinical trials
  - Position papers

Complementary Activities of MC & CTAP

Methodology Committee

- New and updated Methodology Standards
- Dissemination of the Methodology Standards

Advisory Panel on Clinical Trials

- Selection, research design, implementation, technical issues of clinical trials
- Recruitment, Accrual and Retention Subcommittee
- Standardization of Complex Concepts and Their Terminology Subcommittee

Network Research Methods Work Group

- Data Quality and Missing Data expert meeting held on December 10, 2015

- Planned follow up activities include:
  - Potential guidance and standards
  - Webinars and workshops
  - Collaboration with PCORnet

Thank You!

Robin Newhouse, PhD, RN
Chair, PCORI Methodology Committee

Consider for Approval: PCORnet Cross-Patient-Powered Research Networks (PPRN) Demonstration Project

Rachael Fleurence, PhD

Program Director, Research Infrastructure

Purpose

- Patient-Powered Research Networks (PPRNs) have a unique opportunity to broaden the scope of their research to include topics that are meaningful to the larger participant community
- PCORI sought to fund up a comparative effectiveness research (CER) project that will demonstrate scientific, administrative, and operational capacity to collaborate across PPRNs
- This project also had to address comparative clinical and/or health care services questions that reflect shared information needs and decisional uncertainties commonly faced by the collaborating PPRN communities

Project Background

- **Project Title:** Healthy Mind Healthy You
- **Research Question:** What is the comparative effectiveness of two online, evidence-based approaches to using mindfulness to improve well-being?
  - 8 session mindfulness-based cognitive behavioral therapy (MBCT)
  - 3 session “mindfulness light”
- **Study Design:** Prospective Randomized Comparative Effectiveness Trial
  - **Targeted Sample Size:** 8,500
- **Length of follow-up time:** 3 months
- **Total Budget:** $4M
- **All 20 PPRNs are collaborators on the project**
- Led by **MoodNetwork PPRN**

Intervention Background

- **Outcomes:**
  - Well-being (primary), perceived stress, anxiety, depression, psychosocial functioning, quality of life, and mindfulness
- **Specific Aims:**
  - Determine whether a brief 3-session “mindfulness-light” intervention compared to a standard 8-session MBCT intervention will improve well-being in PPRN participants
  - Explore the heterogeneity of treatment effects to both interventions.
  - Contribute to the PCORnet Commons
- **Potential Impact:**
  - Help determine whether a standard MBCT intervention compared to a brief mindfulness approach will have a clinically meaningful effect on individual participant stress and well-being
- **Contributions to the PCORnet Commons:**
  - Web-based intervention tools for managing stress, depression, and anxiety
  - Lessons learned regarding governance, data, engagement, and dissemination for cross-PPRN research

Slate Overview:

Cross-Patient-Powered Research Network (PPRN) Research Demonstration Project

1
New Project

PFA	Allotted	Proposed Total Budget*
Cross-Patient-Powered Research Network (PPRN) Demonstration Project	$4M	$4M

**Total budget = direct + indirect costs*

**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 Cross-Patient-Powered Research Networks (PPRN) Demonstration Project
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

Application Enhancement Efforts

Jean Slutsky, PA, MSPH
Chief Engagement and Dissemination Officer
Program Director for Communication and Dissemination Research

Evelyn P. Whitlock, MD, MPH
Chief Science Officer

Regina L. Yan, MA
Chief Operating Officer

Application Enhancement Efforts (2015-2016)

- 4 workgroups/committees and 1 commissioned external review
- Address concerns from funding announcements through application process, merit review, and feedback to applicants
- ~45 recommendations from across workgroups and external review
- Three overarching principles from the workgroup/committee reports:
  - Ensure PCORI culture supports applicant success
  - Implement change management process
  - Improve and increase communication with external stakeholders

Broad Recommendation Categories

- Letter of Intent (LOI) Review Process
- Application Format
- Application Process
- Merit Review (details to be covered in the future)
- Feedback to Applicants
- Strategic Issues
- Engagement
- Systems Issues
- Training
- Benchmarking

What We've Done So Far on Recommendations

- Organized a staff steering committee of internal stakeholders and decision-makers

- Categorized recommendations for implementation
  - Immediate (Cycle 3 2016 – PFAs post August 2016)
  - Short-term (Cycle 1 2017 – PFAs post February 2017)

- Developing RFP for change management process

Application Enhancement Steering Committee

Goals of the Application Enhancement Steering Committee are to improve:

- Applicant Experience
- Application Quality
- Process Efficiency

Executive Team Sponsors: Evelyn Whitlock, Regina Yan, and Jean Slutsky

Committee Members: Shevonne Polastre, Suzanne Schrandt, Bill Silberg, Scott Solomon, Tsahai Tafari, Dan Tisch, and Kara Walker

Application Enhancement: Immediate by Cycle 3, 2016 – PFAs post August 2016

- Immediately complete a full review of all resource and guidance materials to ensure consistency and reduce duplication
- Enhance the ease of use of resource materials on our website
- Standardize all language and templates
- Move to a single Broad PFA with sections for specific programs
- Improve quality of feedback to applicants
- Develop change management process to review and reduce unnecessary changes

Application Enhancement: Short-term by Cycle 1, 2017 – PFAs post February 2017

- Shorten research plan template
- Review PFA cycle timeline
- Include reviewers and applicants in testing of our online system to improve the user experience
- Implement change management process

Next Steps

- Will continue to work through Science Oversight Committee
- Will provide additional details with future updates

Break

We will return at 1:00 pm ET

Join the conversation on Twitter via #PCORI

Stakeholder Panel: Specialty Physicians

Neil M. Kirschner, Ph.D.

Richard L. Schilsky, M.D., FACP, FASCO

Christopher Ethan Cox, MD

Plans for Dissemination & Implementation at PCORI

Debra Barksdale, PhD, RN
Co-Chair, Engagement, Dissemination, and Implementation Committee

Jean Slutsky, PA, MSPH
Chief Engagement and Dissemination Officer
Program Director for Communication and Dissemination Research

Joanna Siegel, ScD
Director, Dissemination and Implementation

Dissemination and Implementation

- Background
- Initial dissemination and implementation activities for PCORI findings
- Dissemination and implementation activities for selected high-impact studies

Board Discussion to Date:

- Initial presentation to EDIC March 1, 2016
- Updates and discussion at EDIC meeting April 5, 2016

Program Goals

Translation, dissemination, and **implementation** to improve the usability and uptake of research findings, to improve healthcare delivery and health outcomes

- **Translation** … *presentation of research findings in **language and format** that improves their accessibility to and comprehension by the target audience*

- **Dissemination** … *intentional, active process of identifying target audiences and tailoring communication strategies to **increase awareness and understanding of evidence**, and to **motivate its use** in policy, practice, and individual choices (Mathematica Framework 2015)*

- **Implementation** … *deliberate, iterative process of **integrating evidence** into policy and practice through adapting evidence to different contexts and facilitating behavior change and decision making (Brownson et al. 2012)*

Authorizing Legislation

"The purpose of the Institute is to **assist patients, clinicians, purchasers, and policy-makers in making informed health decisions** by advancing the quality and relevance of evidence concerning the manner in which diseases, disorders, and other health conditions can effectively and appropriately be prevented, diagnosed, treated, monitored, and managed **through research and evidence synthesis...**

... and the dissemination of research findings with respect to the relative health outcomes, clinical effectiveness, and appropriateness of the medical treatments, services..."

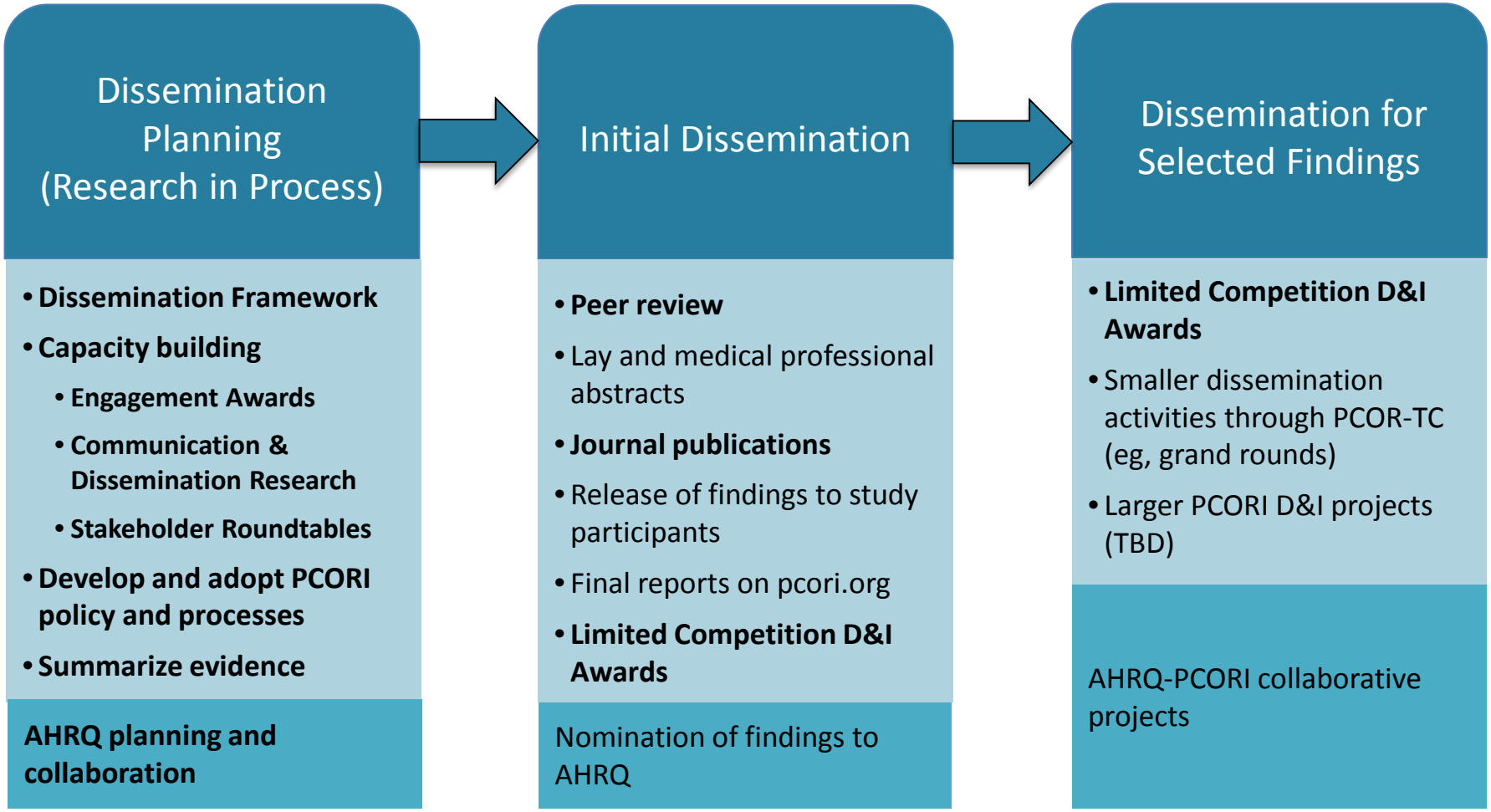
-- from PCORI's authorizing legislation

Authorizing Legislation

"(1) DISSEMINATION.— …the Agency for Healthcare Research and Quality, in consultation with the NIH shall **broadly disseminate the research findings that are published by the Patient Centered Outcomes Research Institute** …. And other government-funded research relevant to comparative clinical effectiveness research. The Office shall create informational tools that organize and disseminate research findings for physicians, health care providers, patients, payers, and policy makers."

-- from PCORI's authorizing legislation

PCORI Dissemination & Implementation Program



Bold type shows activities currently underway

Capacity Building for Dissemination & Implementation

Engagement Roundtables

- Linkages with critical intermediaries for dissemination
- Convening groups of physicians, nurses, purchasers, pharmacy benefit managers
- Highlight opportunities for involvement; identify motivated individuals/ orgs. for future input

Engagement Awards

- Build organizational capacity for dissemination
- Develop and demonstrate the processes, collaborations, and approaches that will facilitate the dissemination to organization's membership or target audience

Communication and Dissemination Research

- Comparative effectiveness of approaches

Initial Dissemination Activities for Findings from PCORI-Funded Studies

Peer Review and Release: PCORI's Obligations

Conduct Peer Review of Primary Research

- Assess scientific integrity
- Assess adherence to PCORI's Methodology Standards

Release Research Findings

- No later than 90 days after "conduct or receipt"
- Make available to clinicians, patients, and general public
- Make comprehensible and useful to patients and providers for healthcare decisions
- Include considerations specific to certain sub-populations, risk factors, and comorbidities
- Describe process and methods, including conflicts of interest; include limitations and further research needed

-- from PCORI's authorizing legislation

Lay and Clinician Abstracts

- "PCORI will create a standardized summary of the study's results for patients and general public….
- Creating and posting the 500-word (lay) abstract … addresses the following specific provision of the law's section "Release of Research Findings": "...(i) convey the findings of research in a manner that is comprehensible and useful …"
- "…no longer than 90 days after PCORI's acceptance of the final research report...PCORI will post on its website the 500-word public-facing summary, the 500-word abstract for medical professionals…."

-- PCORI's Process for Peer Review of Primary Research and Public Release of Research Findings; adopted by the Board of Governors February 24, 2015

Timeline: Primary Completion to Results Posted

Primary completion date—Date of last data collection for the primary outcome

Awardee completes data analysis and prepares draft final research report

Within 13 months, awardee submits draft final research report to PCORI. PCORI initiates peer review. *Note: PCORI strongly encourages awardees to submit their reports promptly*

Within 2 months, PCORI provides peer review comments to awardee

Within 1.5 months, awardee responds with disposition of comments and submits final version of research report. PCORI accepts final research report

Within 3 months, results (clinician and lay-language abstracts) are posted on PCORI.org

Note: PCORI may allow additional time for response to peer review comments.
-- Adopted by the Board of Governors February 24, 2015

Dissemination & Implementation Activities: All funded studies

Translation, Communication

- Lay and Clinician Abstracts; Peer Review Summary
- Investigator journal publications
  - Public Access provisions
- Return of research results to study participants
  - *"PCORI will supply the Awardee Institution with a copy of the 500-word summary … for distribution to study participants and partners. …"*

    -- PCORI's Process for Peer Review of Primary Research and Public Release of Research Findings
- Other - academic presentations, CE/CME, blogs, materials targeting specific audiences

Patient-Centered Outcomes Research – Translation Center

Responsibilities to include:

- **Translation**
  - Writing Lay and Clinician Abstracts
  - Summarizing Peer Review comments
  - Revising PCORI website Project Summaries
  - Developing consistent formats for these products
- **Dissemination activities for selected findings**
  - Grand Rounds, Author in the Room
- **Review of PCORI awards to identify high-impact findings**
- **Projected start date:** July 2016

Public Access to Published PCORI Research Findings

- Policy to improve public access to findings in peer-reviewed literature
- PCORI Awardees will deposit manuscripts in PubMed Central
  - Final, peer-reviewed version of accepted manuscript
  - PubMed Central makes available **6-12 months** after publication depending on journal policy
- To facilitate **immediate access**, PCORI will pay up to $3500 per project, directly to journal, to cover fees for providing free public access upon publication.
  - Journal article must present primary research findings
  - Awardees retain discretion in journal choice

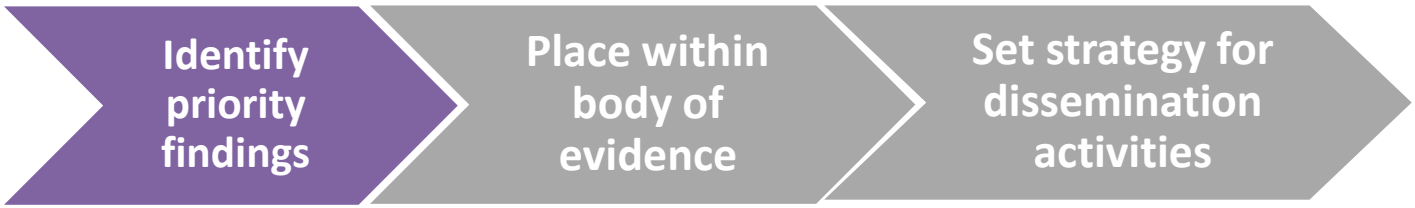
Limited Competition Dissemination and Implementation Awards

Key Information	
Cycle: Cycle 1 2016	
Full Announcement: Dissemination and Implementation of Patient-Centered Outcomes Research Institute (PCORI) funded Patient-Centered Outcomes Research (PCOR) Results and Products in Real-World Settings	**Purpose:** Offer PCORI awardee teams an opportunity to propose ***investigator initiated*** strategies for disseminating and implementing their research results and products.
Letter of Intent (LOI) Deadline: March 2, 2016	**Eligibility:** Current Awardee**;** draft final research report submitted
Application Deadline: June 6, 2016	**Total Direct Costs:** $300,000
Merit Review: September 2016	**Funds available up to:** $2,000,000 per cycle
Awards Announced: November 2016	**Maximum Project Period:** 2 years
Earliest Start Date: January 2017	**Cycles per year:** 3

Dissemination and Implementation Activities for Selected High-Impact Studies

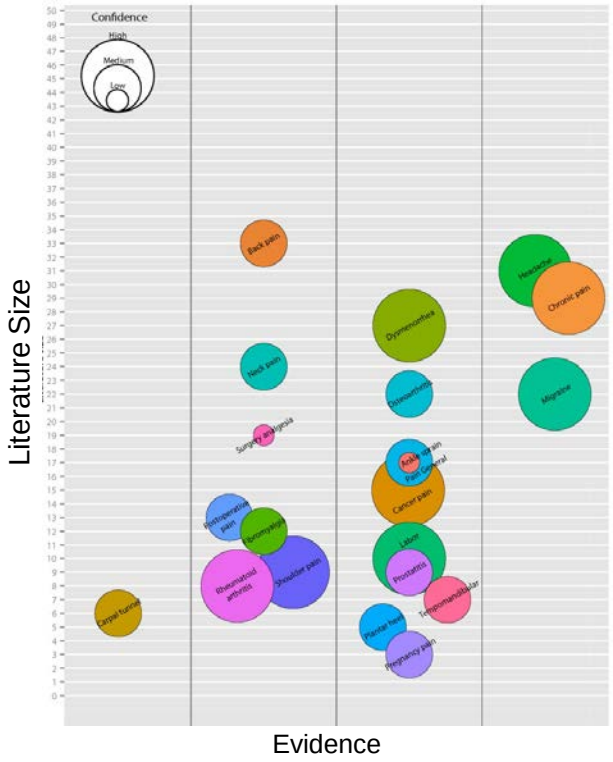
Identify priority candidates for dissemination and implementation efforts

- Targeted PFA topics; Pragmatic Clinical Studies (PCS)
- Broad Awards: Developing processes for peer reviewers, PCOR-TC, others to flag promising results



Summarize body of evidence

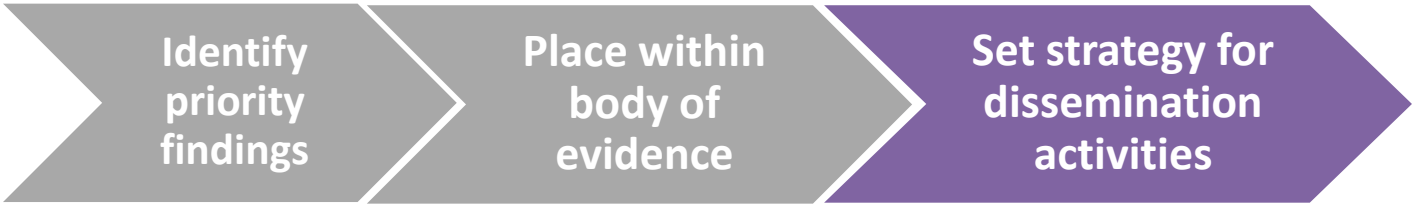
- Evidence summaries available for many topics
- Evidence Mapping



Identify priority findings → **Place within body of evidence** → Set strategy for dissemination activities

Set strategy for dissemination and implementation activities

- AHRQ Dissemination & Implementation of PCOR
  - AHRQ is developing nomination process
  - Will select findings based on strength of evidence, implementation feasibility, other criteria
  - PCORI will submit findings through AHRQ process
  - Multiple opportunities for collaboration in dissemination and implementation initiatives



Future Dissemination & Implementation Efforts

- In collaboration with / complementing AHRQ efforts
- Tailored to specific high-impact findings; topic-specific activities
- Stakeholder-informed as to best approaches
- Awardee teams with strengths and experience in dissemination and implementation
- Multi-pronged dissemination and implementation, large investments for blockbuster findings

Questions, Comments?

Break

We will return at 3:15 pm ET

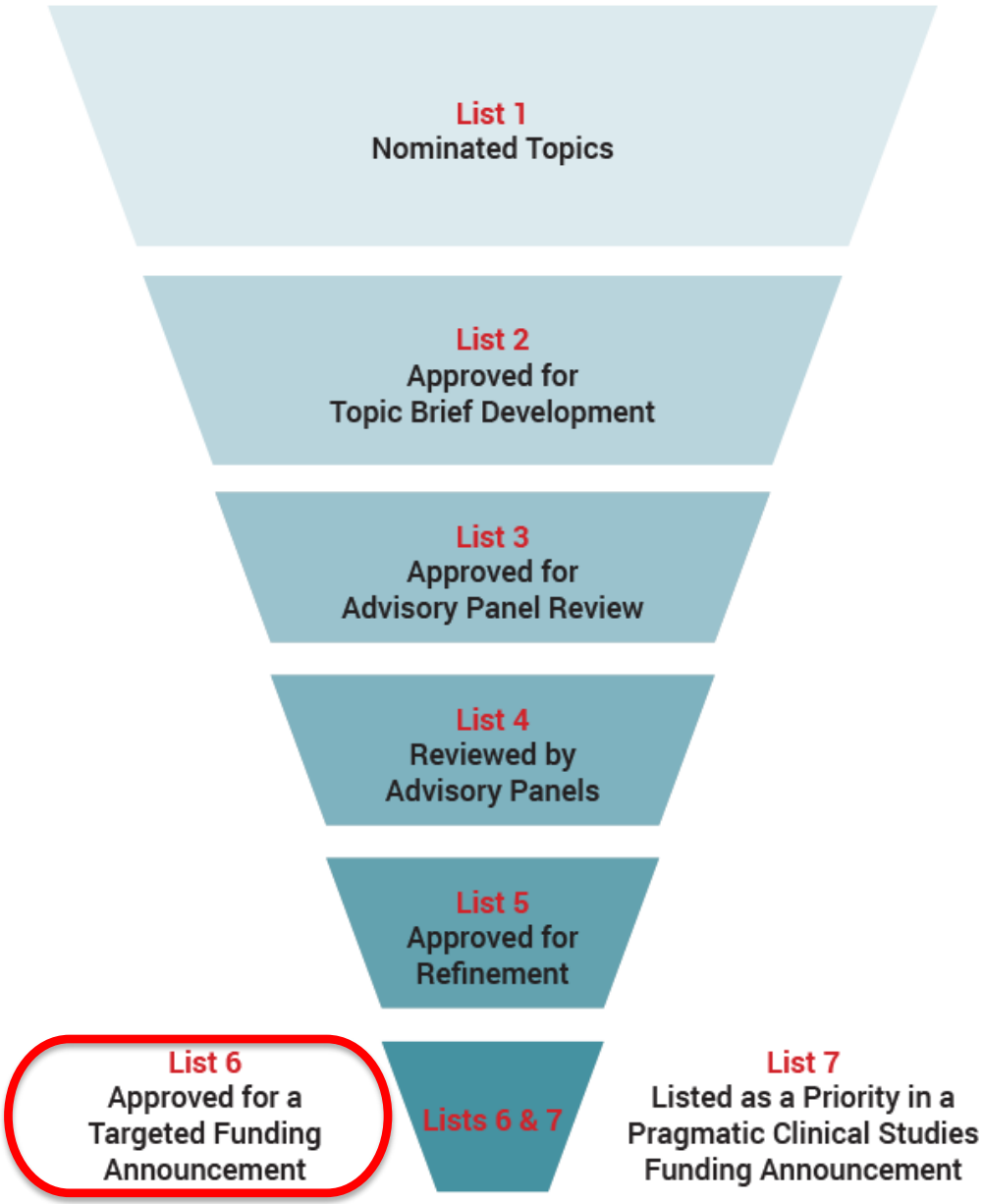
Join the conversation on Twitter via #PCORI

Targeted PCORI Funding Announcement Recommendations for Development

Robert Zwolak, MD, PhD
Science Oversight Committee Chair

Evelyn P. Whitlock, MD, MPH
Chief Science Officer

PCORI Topic Prioritization Pathway



8 Awarded Targeted PFAs to Date

Title	Date Awarded	# of Projects	$ Awarded	Approximate Completion Date
Treatment Options for African Americans and Hispanics/Latinos with Uncontrolled Asthma	December 17, 2013	8	$23	Q2—2017
Treatment Options in Uterine Fibroids (Administered by AHRQ)	September 30, 2014	1	$20	Q4—2019
The Effectiveness of Transitional Care	September 30, 2014	1	$15	Q1—2017
Clinical Trial of a Multifactorial Fall Injury Prevention Strategy in Older Persons (Administered by NIA)	June 4, 2014	1	$30	Q4—2018
Obesity Treatment Options Set in Primary Care for Underserved Populations	September 30, 2014	2	$20	Q2—2018
Optimal Maintenance Aspirin Dose for Patients with Coronary Artery Disease	May 4, 2015	1	$14	Q4—2018
Testing Multi-Level Interventions to Improve Blood Pressure Control in High-risk Populations (Administered by NHBLI)	September 4, 2015	2	$25	Q4—2020
Clinical Management of Hepatitis C Infection	September 28, 2015	2	$39	Q2—2021

5 Approved Targeted PFAs

Title	Expected Award Date	# of Projects	Budget
Treatment-Resistant Depression	Summer 2016	Up to 3	Up to $30M
New Oral Anticoagulants	Summer 2016	Up to 3	Up to $30M
Treatment Strategies for Managing and Reducing Long-Term Opioid Treatment for Chronic Pain	Summer 2016	Up to 4	Up to $40M
Treatment of Multiple Sclerosis	Summer 2016	Up to 8	Up to $50M
Management of Chronic Low Back Pain	Winter 2017	Up to 2	Up to $22M

Targeted PFA Topics for Development Pending Approval

- For Board Vote Today:
  - **Management of Sickle Cell Disease**
  - **Strategies to Prevent Unsafe Opioid Prescribing in Primary Care among Patients with Acute or Chronic Non-cancer Pain**
  - **Community-based Palliative Care Delivery for Adult Patients with Advanced Illnesses and their Caregivers**

Action	Date
Board of Governors Vote on PFA Development	May 23, 2016
Targeted PFA Announced	August 15, 2016
Letter of Intent Due	September 14, 2016
Application Deadline	December 19, 2016
Merit Review	March 27, 2017
Board of Governors Vote to Approve Awards	May 2017

Management of Sickle Cell Disease

Targeted PFA Goal

The goal of the proposed targeted PFA is to generate evidence to:

- Support care transitions from pediatric to adult health care in emerging adults with sickle cell disease (SCD)

Overview: Sickle Cell Disease (SCD)

- SCD is a chronic genetic disorder affecting the body's red blood cells and induces a series of disease-related complications, such as acute chest syndrome, pain crises, and stroke
- Between 70,000-100,000 Americans, predominantly African Americans, have SCD (concentrated in the South and East)
  - Early onset disease (5-6 months of age)
  - Average lifespan ranges between 36 and 56 years
  - **The emerging adult population (ages 16-25) is particularly vulnerable to worsened health outcomes during the time of transition from pediatric to adult care**
- By age 45, SCD patients average ~150 hospital visits, and will have accrued almost $1 million in medical expenses

Care Transitions in Emerging Adults

- **For emerging adults with SCD, transition in care is a life-changing** and continuous process
  - Very different from traditional transition models (e.g., from hospital to home)
  - Children with SCD are now living into adulthood, thus the burden of SCD-related morbidity and mortality has shifted to emerging adults
    - High rates of comorbid conditions (e.g., asthma, restrictive lung disease, cardiac dysfunction and renal dysfunction)
    - Cumulative disease effects

Care Transitions in Emerging Adults (cont.)

- **Quality of care decreases from pediatrics to adult care**
  - Challenges with access to specialists (e.g., hematologists)
    - **~60%** on Medicaid; limits access to specialists
  - Adult care clinicians report **dissatisfaction** with the quality of care they can provide
  - Patients report **dissatisfaction** with quality of care they receive

- **Emerging adults become disengaged from the healthcare system**
  - Loss of usual source of care
    - Decrease in routine preventative and screening visits (for chronic blood transfusions, hydroxyurea treatments, vaccines)
    - More likely to seek care for acute medical events in emergency department
      - **5.0** emergency department visits per year vs. **3.3** in other SCD age groups

Potential to Leverage NHLBI and PCORnet

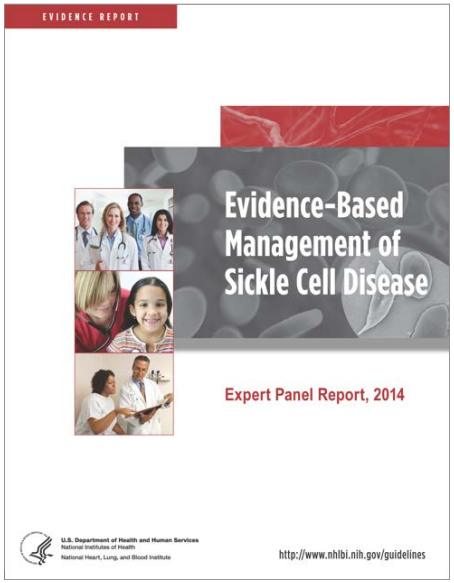
- Targeted PFA can be actively distributed within soon-to-be-funded NHLBI SCD research consortia

- Applicants may potentially collaborate with, and access data from, PCORnet (CDRNs) across the SCD cohorts
  - Three CDRNs have already collected data on 3000+ SCD patients

Collaboration with NHLBI or PCORnet CDRNs would be encouraged, but not required. All are welcome to apply.

Evidence Gaps: Sickle Cell Disease

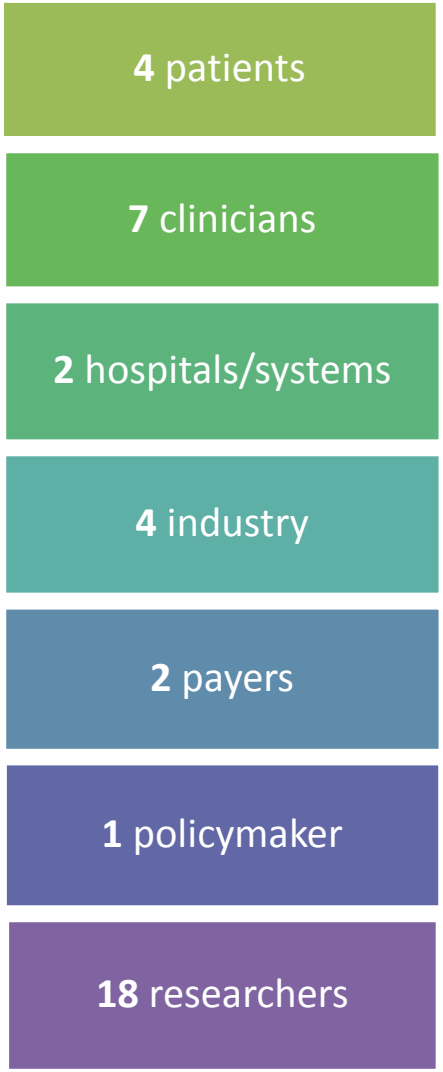
- Current guidelines are based on **weak evidence** and/or **consensus-based opinion**
- SCD-related complications are highest among **emerging adults,** but there is a lack of evidence about how to improve the care transition process and outcomes



- Further research is needed to **help to fill gaps** to improve care processes and outcomes for individuals with SCD
  - There are **no current CER trials** for care transitions for individuals with SCD
    - Necessary to improve healthcare and health outcomes for vulnerable population when evidence base is weak

Summary of Workgroup

- 38 stakeholders submitted **59** questions prior to workgroup meeting
- Staff refined and consolidated the questions into two topic areas*: Care Transitions* and *Pain Management*
- By consensus, each breakout group (care transitions and pain management) identified three potential comparative effectiveness questions, for a total of six potential questions. This PFA focuses on the most important one.



Proposed Research Question & Study Details

- **Research Question:** What is the comparative effectiveness of established transition coordination models for emerging adults with SCD transitioning from pediatric to adult care?

- **Population:** Emerging adults (e.g., 16-25 years of age) with SCD
  - SCD patients typically transition from pediatric to adult care between 16-18 years of age (timing varies based on needs and readiness)
  - Pediatricians may continue to see patients through college
  - By 26 years of age, emerging adults are no longer covered by their parents' insurance
    - Interest in older age (up to 30 years of age) range to assess issues related to insurance transitions for emerging adults

Proposed Research Question & Study Details (cont.)

Interventions and Comparators:

- Interventions must incorporate patients, care givers, and clinicians
- Interventions should be patient-facing, with robust patient engagement
- Direct comparisons of efficacious or commonly used transition coordination interventions
  - Examples could include (but are not limited to):
    - Co-located pediatric and adult care providers;
    - Clinic-based transition coordinator;
    - Virtual consultation (telehealth) with provider or specialist;
    - Use of mHealth (e.g., mobile apps, text messaging)
  - An appropriate comparator may be usual care or standard of care
- Evidence of efficacy in other diseases (e.g., diabetes, cystic fibrosis, congenital heart disease) and transition models may be used

Proposed Research Question & Study Details (cont.)

- **Outcomes:**
  - Health related quality of life (e.g., physical and mental health), depression, patient activation/self-management, patient satisfaction and experiences of care, social functioning (e.g., missed days from work and school)
  - Number of hospitalizations and number of days hospitalized due to complications (e.g., pain crises, strokes, comorbid conditions), measures of emergency department use
- **Study Design:** Cluster RCT with sufficient sample size and/or clusters to power study
- **Setting(s):** Outpatient settings including primary care practices, patient-centered medical homes, specialty SCD clinics
- **Timing:** Maximum 5 year study
- **Proposed Research Commitment:** Up to 3 studies, $25M (total costs)

Board Vote

Call for a Motion to:	• **Approve** $25M (total costs) for Management of Sickle Cell Disease targeted PFA development
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

Strategies to Prevent Unsafe Opioid Prescribing in Primary Care among Patients with Acute or Chronic Non-cancer Pain

Targeted PFA Goal

The goal of the proposed targeted PFA is to generate evidence to:

- Prevent unsafe opioid prescribing while ensuring adequate pain management using either of two related intervention strategies:
  - payer or health system strategies
  - patient and provider communication interventions addressing benefits and harms of various treatments

Background—related PFA, October, 2015

- **Clinical Strategies for Managing and Reducing Long-term Opioid Use for Chronic Non-Cancer Pain Targeted PFA**
  - Among patients with chronic non-cancer pain on moderate/high-dose long-term opioid therapy, what is the comparative effectiveness of strategies for reducing/eliminating opioid use while managing pain?
  - Among patients with chronic non-cancer pain on moderate/low-dose long-term opioid therapy, what is comparative effectiveness and harms of strategies used to limit dose escalation?
  - $40 million for up to 4 awards
- **Awards anticipated July 2016**
- **This proposed announcement is complementary**

Abundance of Evidence Gaps

- Wide variation among states in opioid prescribing rates; indicating a lack of consensus about when to prescribe opioids (CDC, 2016)
- Little evidence exists on how to prevent unsafe prescribing of opioids; research focus largely on patients on chronic opioid therapy (Dy et al, 2016)
  - No systematic reviews, RCTs, or controlled observational studies addressing the effects of opioid prescribing policies on clinical outcomes (Chou et al., 2009)
  - A number of strategies targeted to providers and/or patients to promote safe opioid prescribing have been developed but not rigorously evaluated (HHS, 2014)
  - Strategies that have proven successful in managing chronic pain and reducing the risk of opioid misuse for chronic pain have not been tested to promote safer initiation of opioids (Chang, et al. 2015)
- Guidelines recommend use only when alternatives are ineffective (CDC, 2016; Dy et al., 2016)

Two Research Questions for Targeted PFA

Question 1: What is the comparative effectiveness of **different payer or health system strategies** that aim to prevent unsafe opioid prescribing while ensuring access to non-opioid methods for pain management with the goal of reducing pain and improving patient function and quality of life outcomes, while reducing patient harm?

Question 2: What is the comparative effectiveness of **different patient and provider facing interventions** that facilitate improved knowledge, communication and/or shared decision making about the harms and benefits of opioids and alternative treatments on prevention of unsafe prescribing and improved patient outcomes?

Research Question 1: Payer/Health System Strategies

- **Research Question:** What is the comparative effectiveness of different payer or health system strategies that aim to prevent unsafe opioid prescribing while ensuring access to non-opioid methods for pain management with the goal of reducing pain and improving patient function and quality of life outcomes, while reducing patient harm?
- **Population: Potential new users of opioids** or patients who have **used opioids for < 3 months** with **either acute or chronic pain**. Outside of end-of-life care. Does not include treatment for active cancer.
  - Patients with risk factors for dependence, abuse, and harm
  - Conditions where safer alternatives may be as or more effective
  - Conditions at risk of becoming chronic (e.g., nonstructural low back pain)
- **Interventions:** Must include interventions to prevent unsafe prescribing while ensuring adequate or improved pain management. Interventions must be evidence based or in widespread use.

Research Question 1 (cont.)

- **Outcomes:**
  - **Primary:** Pain, quality of life, functional outcomes, reduction in unsafe prescribing
  - **Secondary:** Anxiety/depression, sleep, disability, harms (tolerance, dependence, addiction/opioid use disorder, overdose, death), provider satisfaction, provider self-efficacy, emergency department utilization
- **Study Design:** Cluster RCT (encourage two active comparators plus usual care arm); or large, prospective observational study; encourage mixed methods
- **Setting:** Primary care, broadly defined to include primary care practices, emergency departments, dentists offices, urgent care centers
- **Time:** 3 years
- **Sample Size**: 600+ for cluster RCT
- **Proposed Research Commitment:** Up to 3 studies, up to $15M (total costs)

Research Question 2: Improved Knowledge, Communication and/or Shared Decision Making

- **Research Question:** What is the comparative effectiveness of different patient and provider facing interventions that facilitate improved knowledge, communication and/or shared decision making about the harms and benefits of opioids and alternative treatments on prevention of unsafe prescribing and improved patient outcomes?

- **Population: Potential new users of opioids** or patients who have **used opioids for < 3 months** with **either acute or chronic pain**. Outside of end-of-life care. Does not include treatment for active cancer.
  - Patients with risk factors for dependence, abuse, and harm
  - Conditions where safer alternatives may be as or more effective
  - Conditions at risk of becoming chronic (e.g., nonstructural low back pain)

Research Question 2 (cont.)

- **Interventions:**
  - Must include interventions to prevent unsafe prescribing while ensuring adequate or improved pain management
  - Must be evidence based or in widespread use
  - May include combinations of patient and provider education, psychological management strategies, and/or self-management strategies
  - Encourage two active comparators but dependent on interventions selected
- **Outcomes:**

	Primary Outcomes	Secondary Outcomes
Patient	- Knowledge - **Patient anxiety (from potential health outcomes)** - **Quality of life (including pain control)** - **Functional outcomes**	- Decisional regret - Satisfaction - Patient involvement preference - **Harms (tolerance, dependence, addition/opioid use disorder, overdose, death)**
Provider	- Rate of opioid initiation - **Reduction in unsafe prescribing** - Repeat opioid prescriptions - Knowledge	- **Satisfaction** - Length of visit - Confidence and **self-efficacy**

Research Question 2 (cont.)

- **Study Design:** RCT or cluster RCT
- **Setting:** Primary care, broadly defined to include primary care practices, emergency departments, dentist offices, urgent care centers
- **Time:** 3 years
- **Sample Size:** 1700 or greater with multiple follow-up data collection points
- **Proposed Research Commitment:** 3-5 studies, up to $15M (total costs)

Board Vote

Call for a Motion to:	• **Approve** $30M (total costs) for Preventing Unsafe Opioid Prescribing targeted PFA development
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

Community-based Palliative Care Delivery for Adult Patients with Advanced Illnesses and their Caregivers

Targeted PFA Goal

The goal of the proposed targeted PFA is to generate evidence to:

- Support **care planning over time** that is consistent with the goals and preferences of patients with advanced illnesses and their caregivers, and
- Support the **delivery of coordinated, community-based palliative care** that effectively implements those care plans

Overview

- **Advanced, serious illnesses result in significant patient and caregiver burden** in terms of physical and psychological symptoms and declining quality of life (QOL) (Kelley and Morrison, 2015; IOM 2014)
- **Palliative care makes a difference**
  - Systematic reviews show that patients with advanced illnesses who receive palliative care services report clinically meaningful improvements in QOL, lower symptom burden, lower caregiver distress, and reduced hospitalizations (Dy et al., 2012; ICER, 2016; Gomez et al., 2013)
- **Core components of palliative care** include: (IOM, 2014; NCP, 2013)
  - Systematic assessment and management of patient symptoms
  - Psychosocial support for patients and caregivers
  - Advance care planning
  - Coordination among different clinicians to facilitate goal concordant care
- **Palliative care is more than Hospice**
  - Hospice is a setting for delivering palliative care to individuals nearing death

Stakeholder Perspectives

- **Patients and caregivers:** Access to palliative care services is typically limited to inpatient hospitals or end-of-life hospice settings; patients and caregivers need palliative care where they live – in their community (CAPC, 2015)
- **Clinicians:** Limited workforce of palliative care specialists results in significant strain on meeting the needs of patients and caregivers in the community; community-based clinicians feel underprepared to communicate about and deliver palliative care (CAPC, 2015, Kamal et al., 2013)
- **Health systems and payers:** Several approaches to delivering community-based palliative care are emerging; decision makers need comparative information on the most **effective and efficient** ways of organizing and delivering palliative care in the community (ICER, 2016)
- A **2014 WHO resolution** urges member states to integrate evidence-based palliative care services across all levels of care, **with emphasis on primary care, community and home-based care** (WHO, 2014)

Timeliness of the Targeted PFA

- January 1, 2016 approval of Medicare reimbursement for ACP discussions
- ACA payment reforms incentivize delivery of high quality, coordinated care
- Research funders (e.g., NIH, American Cancer Society) and payers (e.g., CMS) consider PCORI funding of large scale head-to-head trials to be the natural evolution of research in palliative care
- Several federally funded networks and consortia for conducting large scale, multi-site palliative care trials currently exist
- PCORI's multi-stakeholder meeting (82 attendees) identified **advance care planning and community-based models of palliative care** as important priority areas for CER
- Recent reports call upon PCORI and other funders to support research **to facilitate advance care planning (ACP) and identify the optimal mix of providers and settings to deliver coordinated, community-based palliative care** (IOM, 2014; Kelley and Morrison, 2015; CAPC, 2015; Schenker and Arnold, 2015; Halpern, 2015)

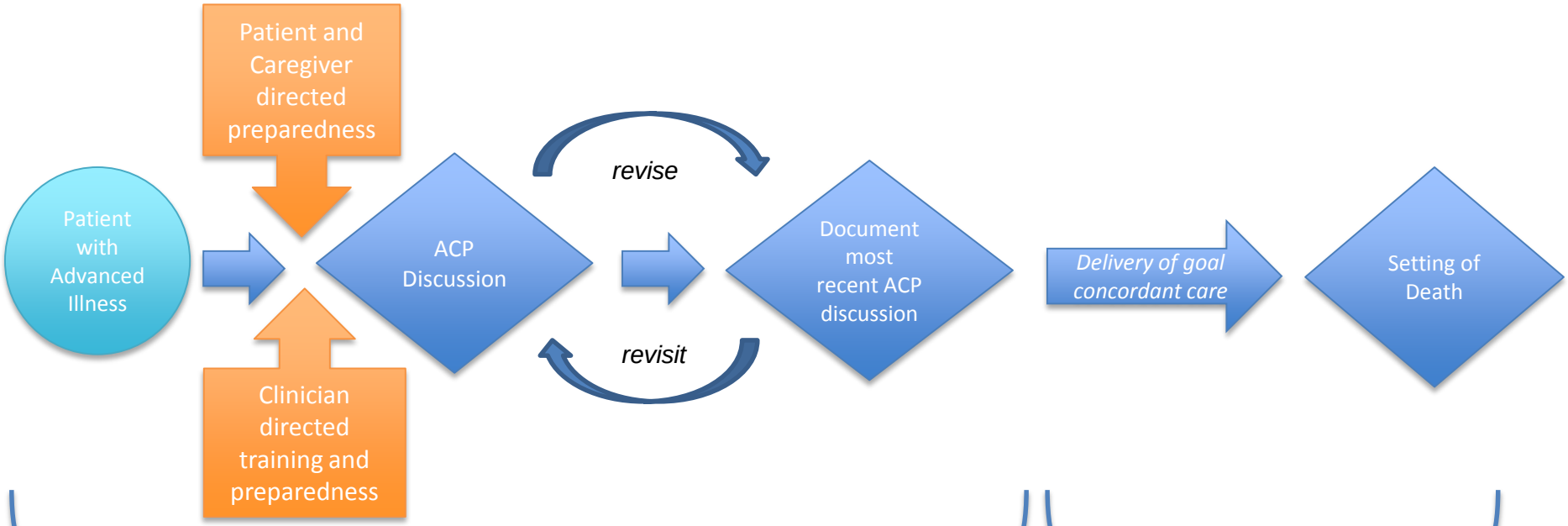
Evidence Gaps Limit the Implementation of ACP

- Most ACP studies:
  - Have focused on relatively short-term outcomes and not looked at the impact of ACP on goal concordant care
  - Studies have not focused on facilitating ACP **over time**
  - Most studies have separately looked at either patient-directed interventions or clinician-directed interventions
    - Few studies have evaluated clinician and patient combined interventions
- Systematic reviews suggest:
  - Future studies are needed to understand the effective elements of ACP and the best way to implement structured ACP in standard care (Houben et al., 2014)
  - Randomized studies, in community settings, with a focus on standardized patient and caregiver outcomes are lacking (Brinkmann-Stoppelenburg et al., 2014)

Question 1: Advance Care Planning

- **Research Question:** What is the comparative effectiveness of different patient, caregiver, and clinician-directed and combination approaches to facilitating advance care planning conversations between adult patients living with advanced illnesses, their caregivers, and clinicians on patient-centered and other outcomes over time?
- **Population:** Geographically and racially/ethnically diverse patients living at home with any advanced illness and who experience a high symptom burden and/or functional limitations and their caregivers
  - Example conditions include advanced heart failure, COPD, advanced kidney disease, advanced neurodegenerative diseases, advanced cancers
  - Clinicians providing healthcare to patients with advanced illness
- **Interventions & Comparators:** Efficacious and/or widely used programs and interventions designed to facilitate ACP conversations and documentation of goals of care for patients with advanced illness over time:
  - Approaches directed at patients and caregivers (e.g. question prompt lists)
  - Approaches to support clinicians in delivering ACP (e.g. clinician skills training)
  - Combined approaches (e.g. Respecting Choices program)

Outcomes



Proximal Outcomes:

Process

- Identification of surrogate decision maker
- Identification of goals of care
- Documentation of ACP discussion
- Discussion of goals of care with provider

Patient-centered

- Satisfaction with communication
- Experience with care
- Shared decision making

Intermediate Outcomes:

Process

- Revised ACP documentation
- Frequency of ACP discussion

Patient-centered

- Understanding of prognosis
- Decision satisfaction, decision regret
- Patient and caregiver QOL, distress, burden

Distal Outcomes:

- Goal concordant care
- Setting of death
- Bereavement

Question 1: Advance Care Planning (cont.)

- **Timing:** Up to 5 years
- **Setting:** Multi-site community-based settings such as hospital-based clinics, solo or group physician practices, and the patient's home
  - In-patient institutionalized settings such as nursing homes and hospices are not addressed under this announcement
- **Design:** RCT or cluster RCT, mixed methods are of interest
- **Sample Size:** N= 750+ patient and caregiver dyads; multiple follow-up data collection points
- **Proposed Research Commitment:** 3-5 studies, up to $18M (total cost)

Models of Care: Evidence Gaps

- Efficacious models exist, but the utility of the available evidence is limited for informing decisions about the organization and delivery of palliative care services in community settings: (Gomez, 2013; Dy et al., 2012; ICER, 2016)
  - Multi-site studies are lacking
  - Study sample typically lacks geographical and racial/ethnic diversity; studies rarely have adequate statistical power for subgroups analyses
  - There is need to standardize outcomes across studies to facilitate comparisons
  - Comparator in almost all studies is usual care
- Head-to-head studies are needed

Question 2: Models of Care

- **Research Question:** What is the comparative effectiveness of different established models of palliative care in community settings on improving patient-centered and other outcomes among adult patients with advanced illnesses and their caregivers?
- **Population:** Geographically and racial/ethnically diverse patients living at home with any advanced illness and who experience a high symptom burden and/or functional limitations and their caregivers
  - Example conditions include advanced heart failure, COPD, advanced kidney disease, advanced neurodegenerative diseases, advanced cancers
  - Applications focused on facilitating the delivery of palliative care in rural and other low resources settings are of particular interest
- **Interventions & Comparators:** Palliative care models to be evaluated in a head-to-head comparison may vary on one or more of the following parameters:
  - **Level of integration** between primary/subspecialty clinicians and palliative care specialists (e.g., consultative model, nurse-led case management model, co-management model)
  - **Site** of palliative care delivery (outpatient clinic/office, home, or both)
  - **Method of care delivery** (in person visit, remote/telemedicine, or both)

Question 2: Models of Care (cont.)

- **Outcomes:**
  - **Primary:** Patient and caregiver QOL; patients' symptom burden; patient and caregiver distress, caregiver burden; receipt of goal concordant care
  - **Secondary:** Patient experiences of and satisfaction with care, perceptions of symptoms management; healthcare utilization (hospitalizations, emergency department visits); out of pocket costs/expenses
- **Timing:** Up to 5 years
- **Setting:** Multi-site, community-based settings such as hospital-based outpatient clinics, solo or group physician practices, or the patient's home
  - Inpatient, institutionalized settings such as nursing homes and hospices are not of interest
- **Design:** Cluster RCT
- **Sample Size:** N = 1,000+ patients and their caregivers (total N =2,000)
  - Multiple follow-up data collection points
- **Proposed Research Commitment:** Up to 3 studies, up to $30M (total costs)

Timeline

Action	Date
Advisory Panel	May 27-28, 2015
Multi-stakeholder Workshop	March 7, 2016
SOC Endorsement	April 26, 2016
Board of Governors Vote on PFA Development	**May 23, 2016**
Targeted PFA Announced	August 15, 2016
Letter of Intent Due	September 14, 2016
Application Deadline	December 19, 2016
Merit Review	March 27, 2017
Board of Governors Vote to Approve Awards	May 2017

Board Vote

Call for a Motion to:	• **Approve** $48M (total costs) for Palliative Care targeted PFA development
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

Additional Proposed Studies to 2015 Cycle 1 and Cycle 2 Award Slates

Christine Goertz, DC, PhD
Chair, Selection Committee

Evelyn P. Whitlock, MD, MPH
Chief Science Officer

Additional Study to 2015 Cycle 1 Pragmatic Clinical Studies Slate

2015 Cycle 1 Pragmatic Studies Funding Slate

*1 Additional Recommended Project**

Project Title
Determining the Optimal Treatment Strategy for Patients who have Chronic Migraine with Medication Overuse
RofLumilast or Azithromycin to preveNt COPD Exacerbations (RELIANCE)
Dissemination of Effective Smoking Cessation Treatment to Smokers with Serious Mental Illness
Patient Empowered Strategy to Reduce Asthma Morbidity in Highly Impacted Populations (PESRAMHIP)
Comparison of Operative versus Medical Endocrine Therapy for low risk DCIS: The COMET Trial
Comparative Effectiveness of Breast Cancer Screening and Diagnostic Evaluation by Extent of Breast Density

**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.*

Comparative Effectiveness of Breast Cancer Screening and Diagnostic Evaluation by Extent of Breast Density

- **Research Question(s):** How can **breast cancer screening be personalized** to account for women's differing breast densities? Does **MRI imaging enhance the preoperative evaluation** of an initial breast cancer in women, overall or by their differing breast densities?
- **Population:** Women, aged 18-64, with all degrees of breast density
- **Intervention/Comparison, Aim 1:** Screening digital mammography alone *versus* with supplemental screening (tomosynthesis, ultrasound, MRI) in women, considering also the extent of breast density
  - **Aim 2:** Preoperative breast imaging with MRI *versus* no preoperative MRI in women with initial breast cancers, considering also breast density
- **Added Substudy:** Does community radiologists' interpretation of breast tomosynthesis images change as a result of years of experience or cumulative volume of readings?
  - Does any "learning curve" vary by location of radiologists (academic, community) or specialty focus (general, breast-specialized)?

Comparative Effectiveness of Breast Cancer Screening and Diagnostic Evaluation by Extent of Breast Density

- **Outcomes:**
  - Aim 1: Rates of screen-detected early stage cancers, interval or late-stage cancers, patient-reported outcomes, recall rates, and projected long-term clinical outcomes (breast cancer deaths averted, life years gained, and over-diagnosis)
  - Aim 2: Rates of second cancers, additional cancers detected, definitive surgeries
- **Study Design:**
  - Observational study using with women classified on the basis of imaging studies received, supplemented by surveys and modeling
  - **Sample Size:** 2.8M exams (among 1 million women)
- **Budget:** $8M

Comparative Effectiveness of Breast Cancer Screening and Diagnostic Evaluation by Extent of Breast Density

- **Engagement:** Includes Patient Advisory Boards, and Stakeholder Panel comprised of clinical, advocacy, health system, payer, and policy members
- **Potential Impact:**
  - Limited evidence on how to best incorporate breast density into clinical care, despite the fact that breast density impacts malignancy detection on screening and direct-to-consumer laws notify women of their breast density in many states, raising questions of supplemental screening
  - Limited evidence on whether pre-surgical MRI improves breast cancer outcomes in women, overall or by degree of breast density
  - Understanding whether there is a learning curve for some or all radiologists in their performance with a new technology (tomosynthesis) will enhance the accuracy of this study findings and could inform quality considerations in community practice

Slate Overview – 2015 Cycle 1
Large Pragmatic Studies PFA

Approved 5 Projects

Proposed 5 + 1 = 6 Projects

Pragmatic Studies PFA	Announced	Previously Approved Budget	Proposed Total Budget*
Large Pragmatic Studies to Evaluate Patient-Centered Outcomes	$90M	$59M	$67M

**Total budget = direct + indirect costs*

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.

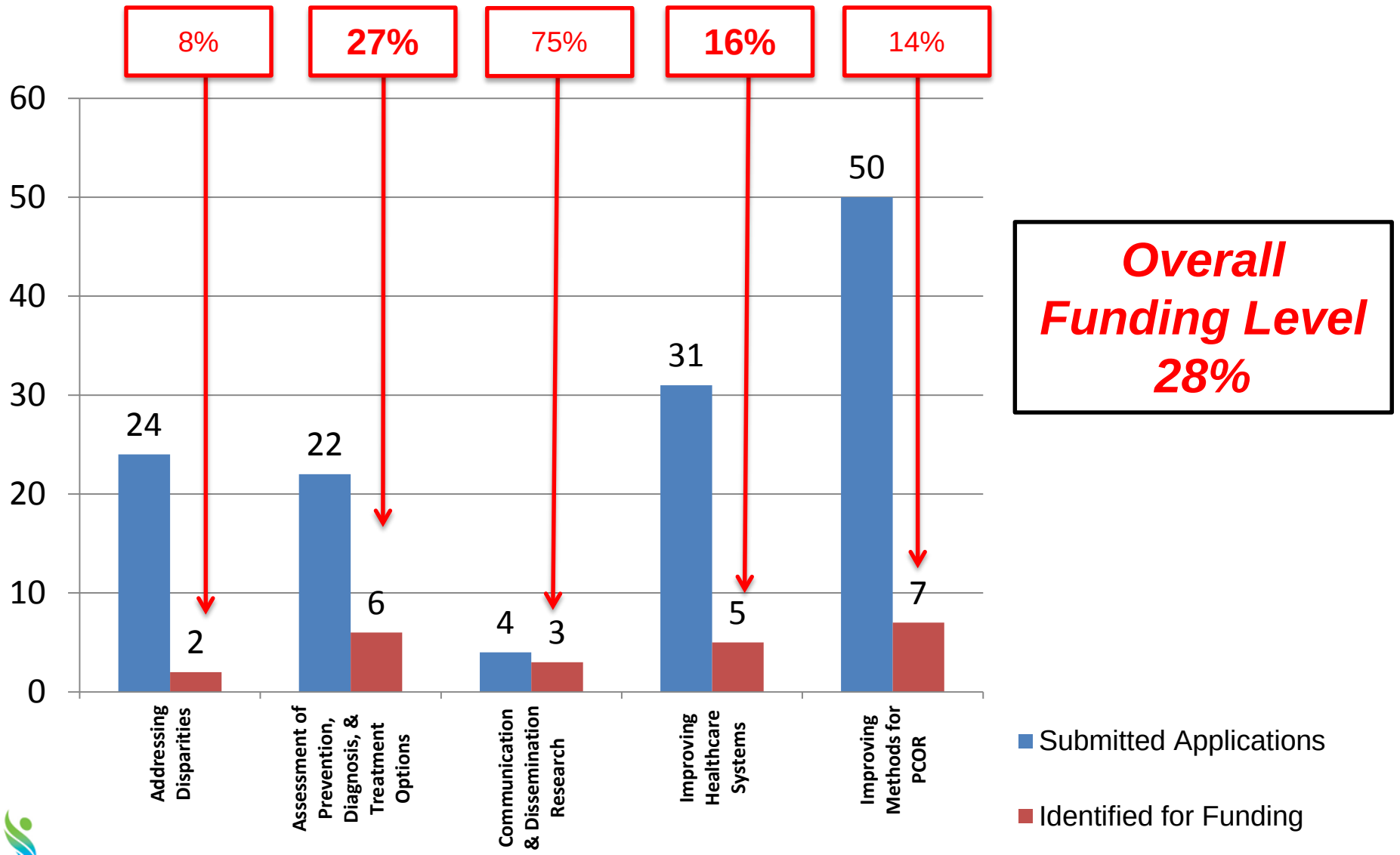
Board Vote

Call for a Motion to:	• **Approve** funding the recommended additional award from the Spring 2015 Pragmatic Clinical Studies Cycle
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

Additional Studies to Broad Cycle 2 2015 Slate

Broad Cycle 2 2015

What Percentage of Applications are We Proposing to Fund?



Overall Funding Level 28%

Assessment of Prevention, Diagnosis, and Treatment Options

*1 Additional Recommended Project**

Project Title
Continued Anticonvulsants after Resolution of Neonatal Seizures: a Patient-centered Comparative Effectiveness Study
Comparative Effectiveness Analyses among Conservative Treatment Strategies For Ductal Carcinoma In Situ
Multi-institutional Trial of Non-operative Management of Uncomplicated Pediatric Appendicitis
Improving Care for Veterans with Post-Traumatic Stress Disorder (PTSD): Comparative Effectiveness of Medications to Augment First-line Pharmacotherapy
Longitudinal Comparative Effectiveness of Bipolar Disorder Therapies
High Intense Periodic vs. Every Week Therapy in Children with Cerebral Palsy (CP)

*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.
Resubmissions in bold.

Improving Healthcare Systems

*1 Additional Recommended Project**

Project Title
Healing through Education, Advocacy and Law (HEAL) in Response to Violence
Patient Osteoarthritis Careplan to Inform Optimal Treatment
Comparing Interventions to Increase Colorectal Cancer Screening in Low-Income and Minority Patients
Pragmatic Trial Comparing Telehealth Care and Optimized Clinic-Based Care for Uncontrolled High Blood Pressure
Patient-Centered Hepatitis C Care via Telemedicine for Individuals on Opiate Substitution Therapy: A Stepped Wedge Cluster Randomized Control Trial

*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. Resubmissions in bold.

Slate Overview – Cycle 2 2015
Broad PFAs

Approved 21 Projects

Proposed 21 + 2 = 23 Projects

Broad PFA	Allotted	Previously Approved Award Budget	Proposed Total Budget*
Addressing Disparities	$8M	$4M	$4M
Assessment of Prevention, Diagnosis, and Treatment Options	$32M	$10M	$13M
Communications and Dissemination Research	$8M	$6M	$6M
Improving Healthcare Systems	$16M	$17M	$24M
Improving Methods for Conducting PCOR	$12M	$8M	$8M
TOTAL:	$76M	$45M	$55M

*Total budget = direct + indirect costs. 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 two additional awards from the 2015 Cycle 2 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
Roll Call Vote:	• Vote to **Approve** the **Final** Motion • Ask for votes in favor, opposed, and abstentions

PCORI Projects with Key Outcomes: Depression, Pain, Sleep, and Fatigue

Evelyn P. Whitlock, MD, MPH

Chief Science Officer

Heather Edwards, PhD

Program Officer, Evaluation & Analysis

Approach

1. Searched portfolio data to identify projects that were coded as having one of the following outcomes:
    - Pain assessments or control – defined as a *reduction in perceived pain*
    - Level of anxiety, depression, mood or well-being – defined as *the level of anxiety, depression, mood, wellbeing of the patient or caregiver (henceforth "Depression")*
    - Coders' notes for *insomnia* or *sleep (henceforth "Sleep Outcomes")*, and *fatigue*

2. Supplemented portfolio data by searching research plans for *pain*, *insomnia*, *sleep*, *fatigue*, and *depression* keywords

3. Noted whether an outcome in one of the four areas appeared to be a primary or secondary outcome of the project, based on a single-coder's assessment

4. Recorded the instrument or tool proposed to measure the outcome

Results

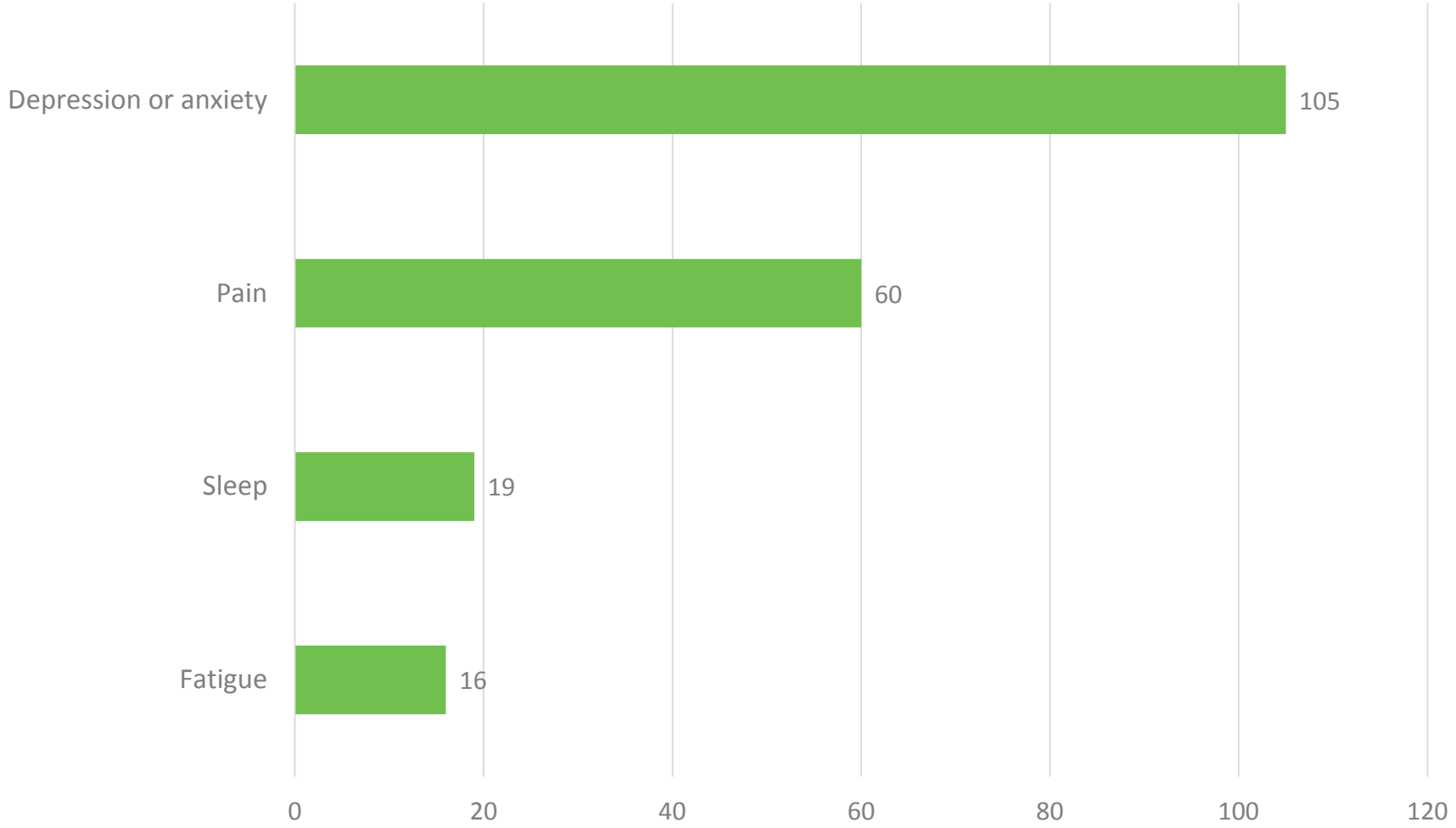
- The search excluded Methods, Pilots, MOUs, PPRN, and CDRN projects, and included 285 other research projects.*

- The process identified 136 research projects that planned to report one or more outcome related to pain, depression or anxiety, sleep issues, or fatigue. Many of these 136 projects included more than one of the outcomes and outcome categories. Specifically, we identified 240 mentions of these four outcomes in the 136 projects.

*Through the Spring 2015 PCS Cycle, awarded September 2015

Number of Projects with Depression, Pain, Sleep, and Fatigue Outcomes

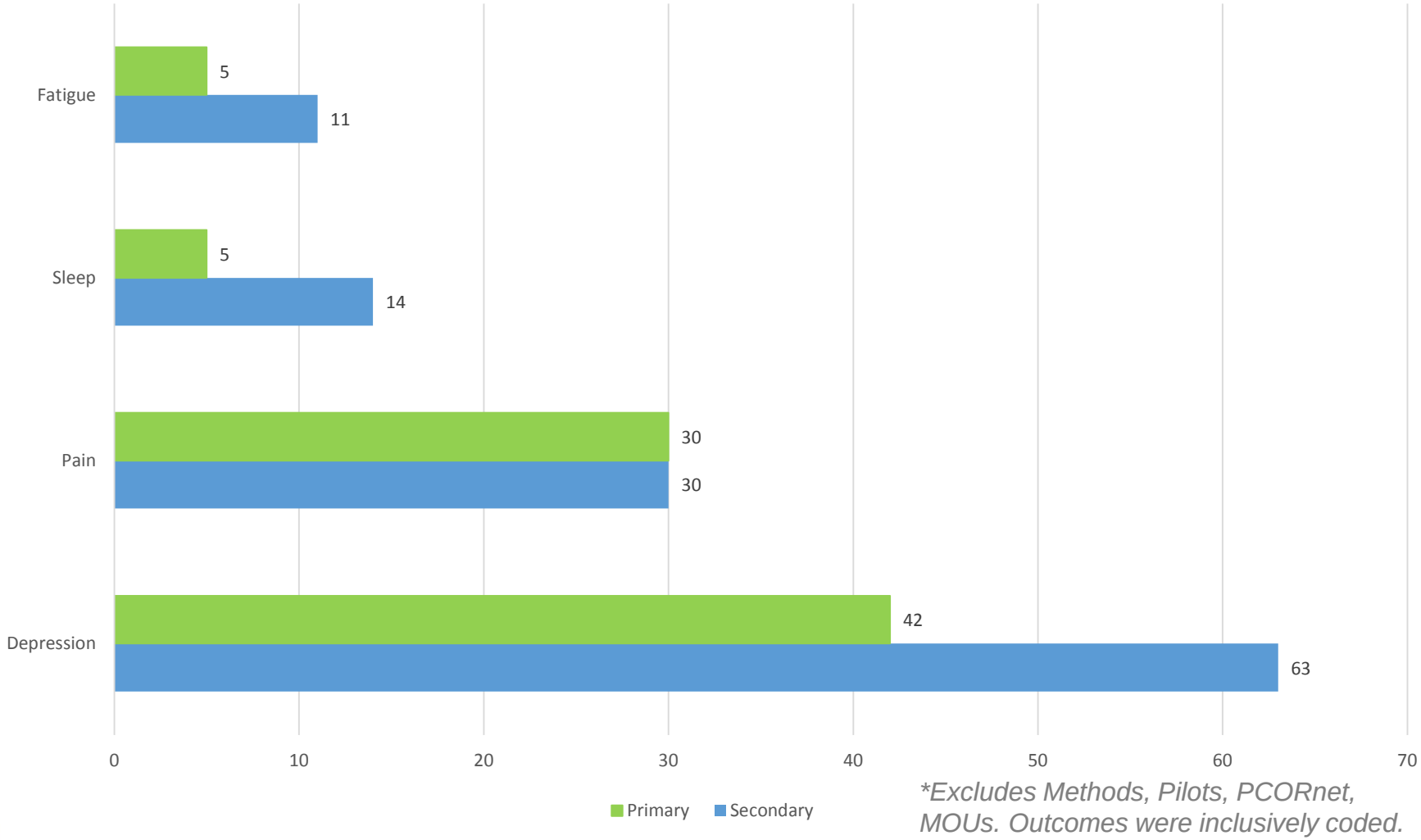
Cycle I-Spring 2015 PCS Cycle (N=136)*



*Excludes Methods, Pilots, PCORnet, MOUs. Outcomes were inclusively coded.

Projects by Primary and Secondary Outcomes

Initial review of 136 projects for outcomes of Pain, Depression or Anxiety, Sleep, and Fatigue: primary outcome for 82 projects; secondary outcomes for 117 projects.



**Excludes Methods, Pilots, PCORnet, MOUs. Outcomes were inclusively coded.*

Conditions

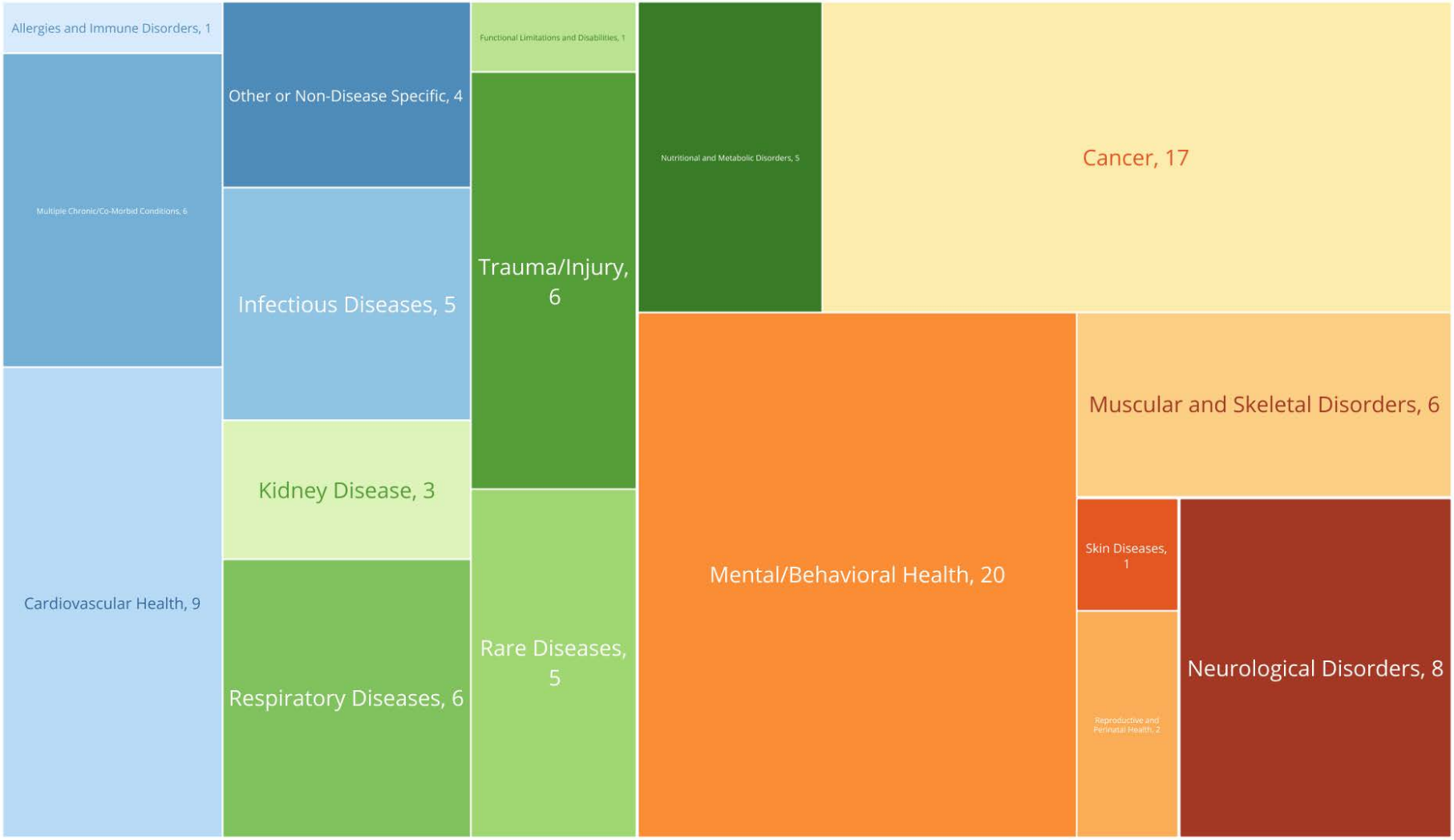
- For each outcome, we counted the primary conditions represented
- Each project is counted once, even if coded as representing more than one condition (i.e. cancer and mental health)

Measures

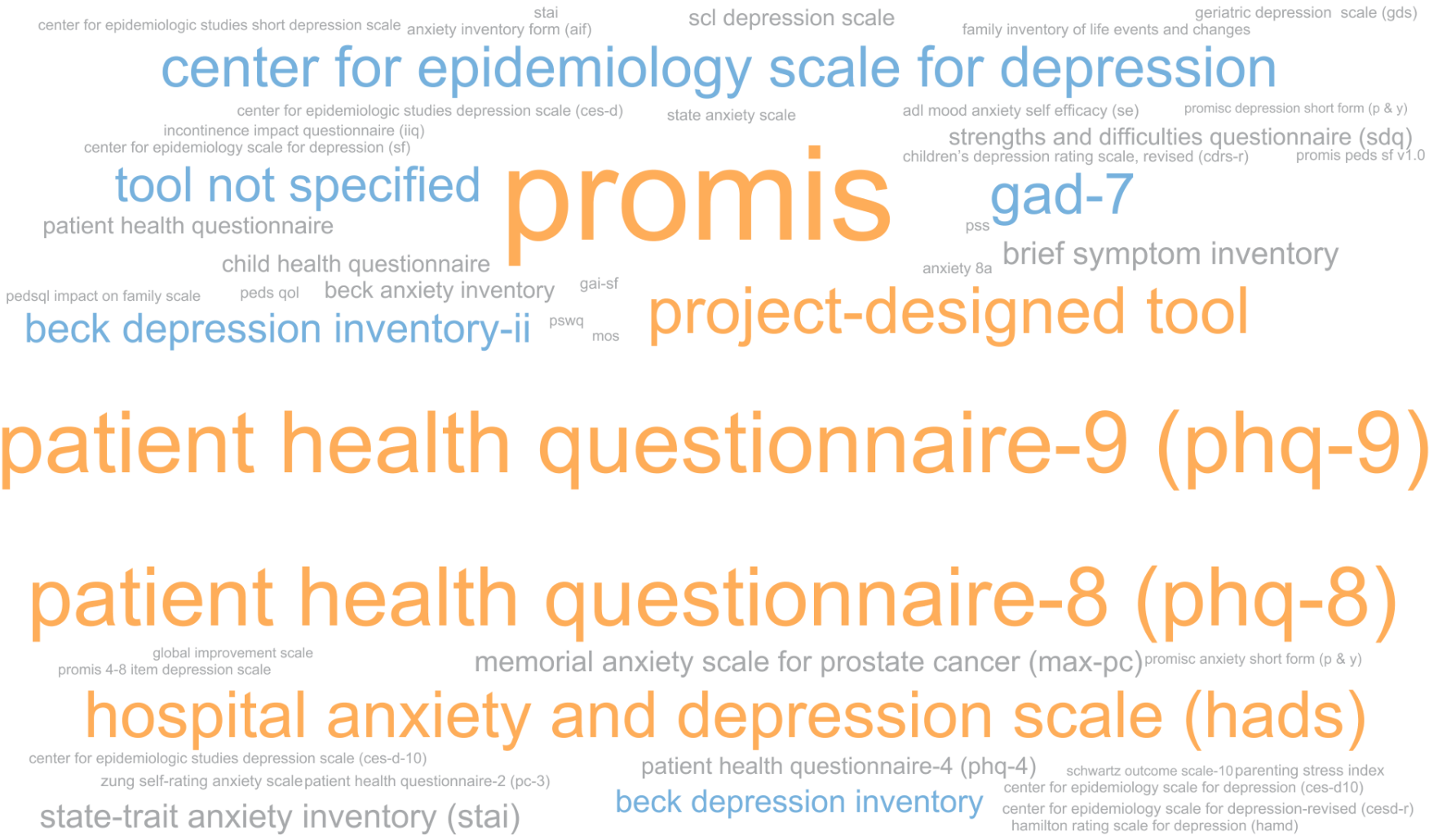
- Measures associated with each outcome are shown as word clouds
- The larger the word in the word cloud, the more projects use that measure of the outcome
- The purpose of this set of exploratory slides was to consider how commonly measurement approaches are being used across projects

Conditions with Depression or Anxiety as an Outcome (N=105 projects)

The size of the rectangles correspond to the number of projects with these conditions.

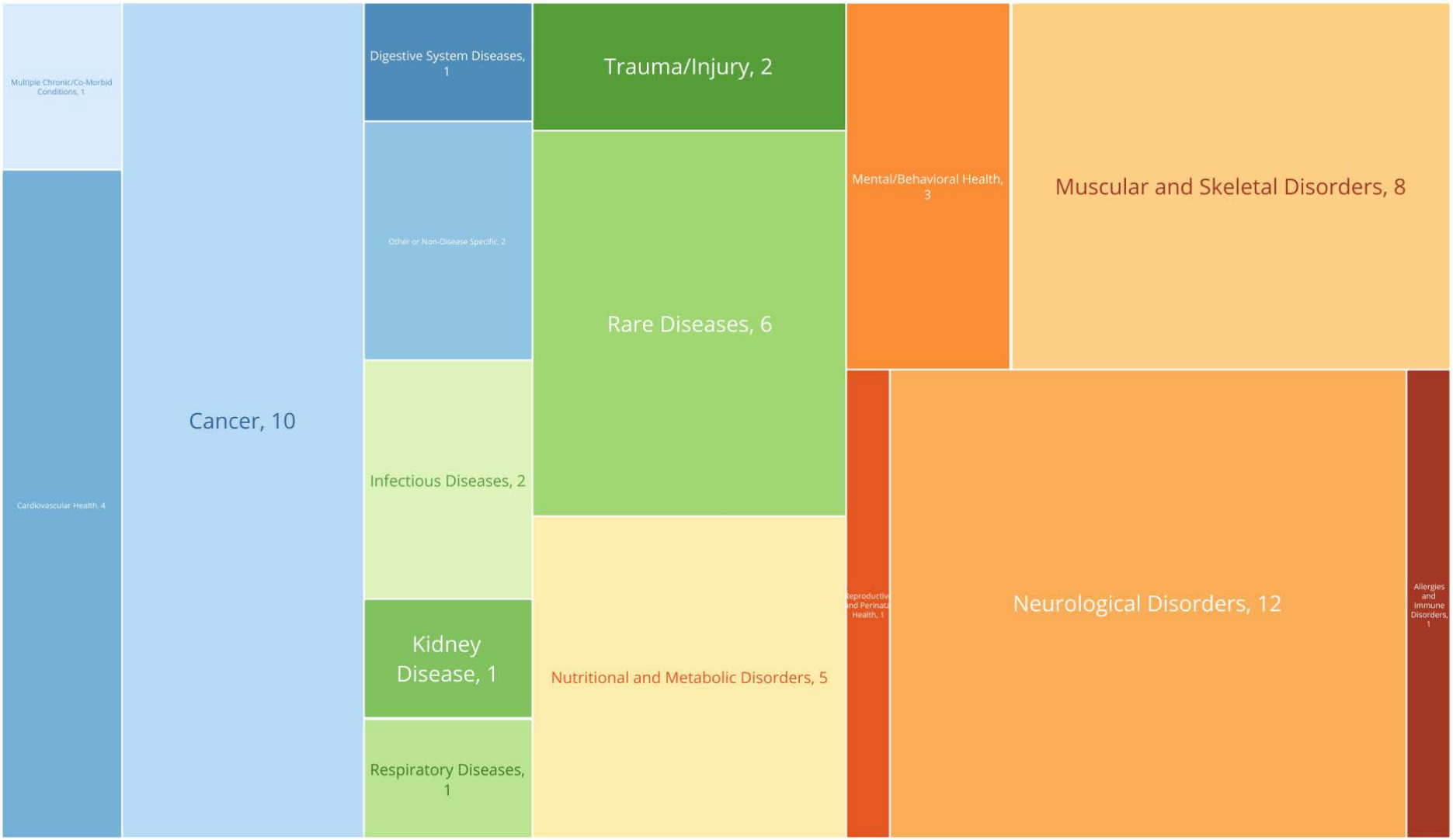


Depression & Anxiety Measures



Conditions with Pain as an Outcome (N=60)

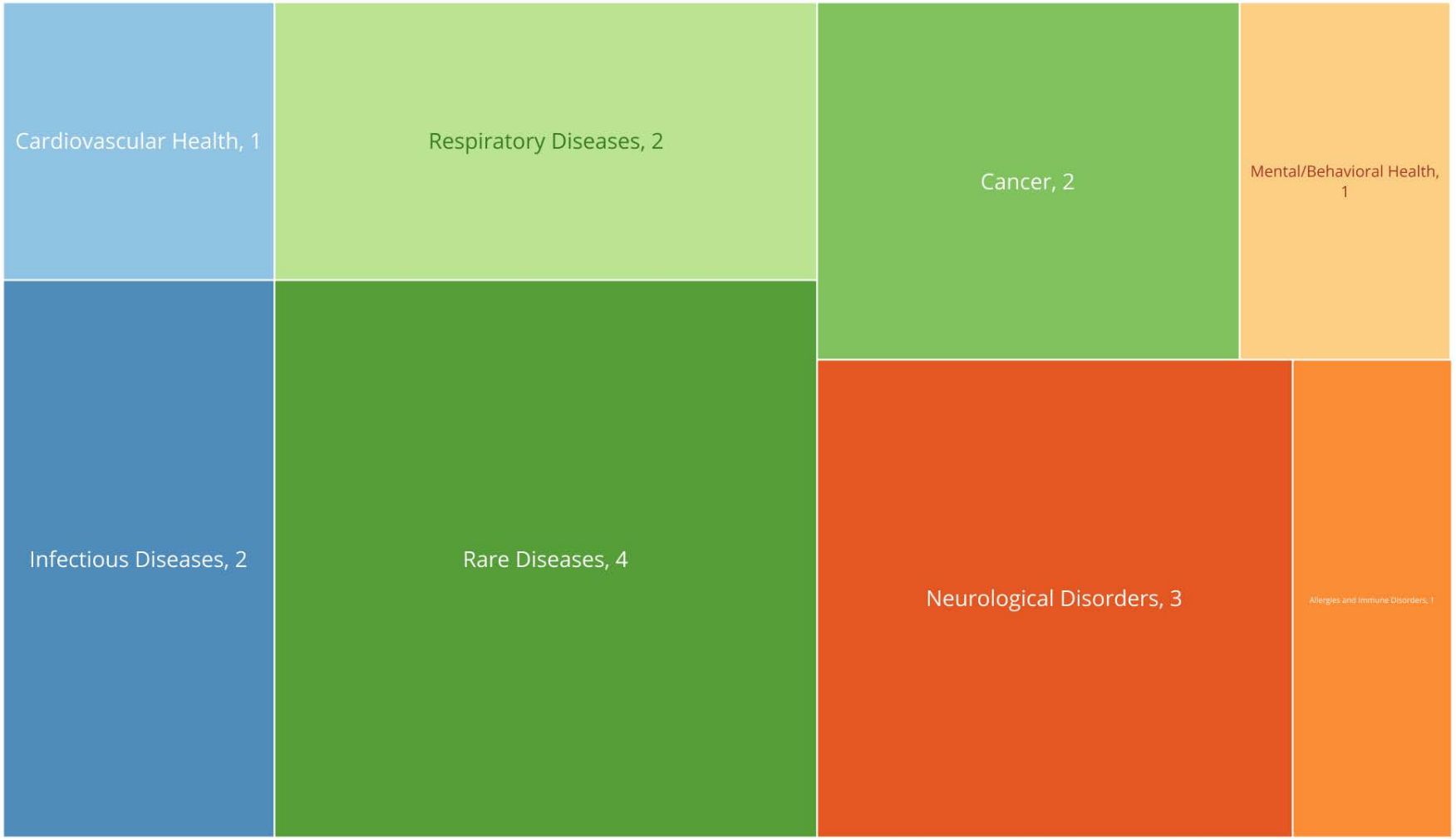
The size of the rectangles correspond to the number of projects with these conditions.



Pain Measures

Conditions with Fatigue as an Outcome (N=16)

The size of the rectangles correspond to the number of projects with these conditions.



Fatigue Measures

memorial symptom assessment scale

fatigue severity scale (fss)

sf-12

sf-36

promis

fatigue severity scale

multidimensional fatigue inventory-short form (mfsi-sf)

hrqol

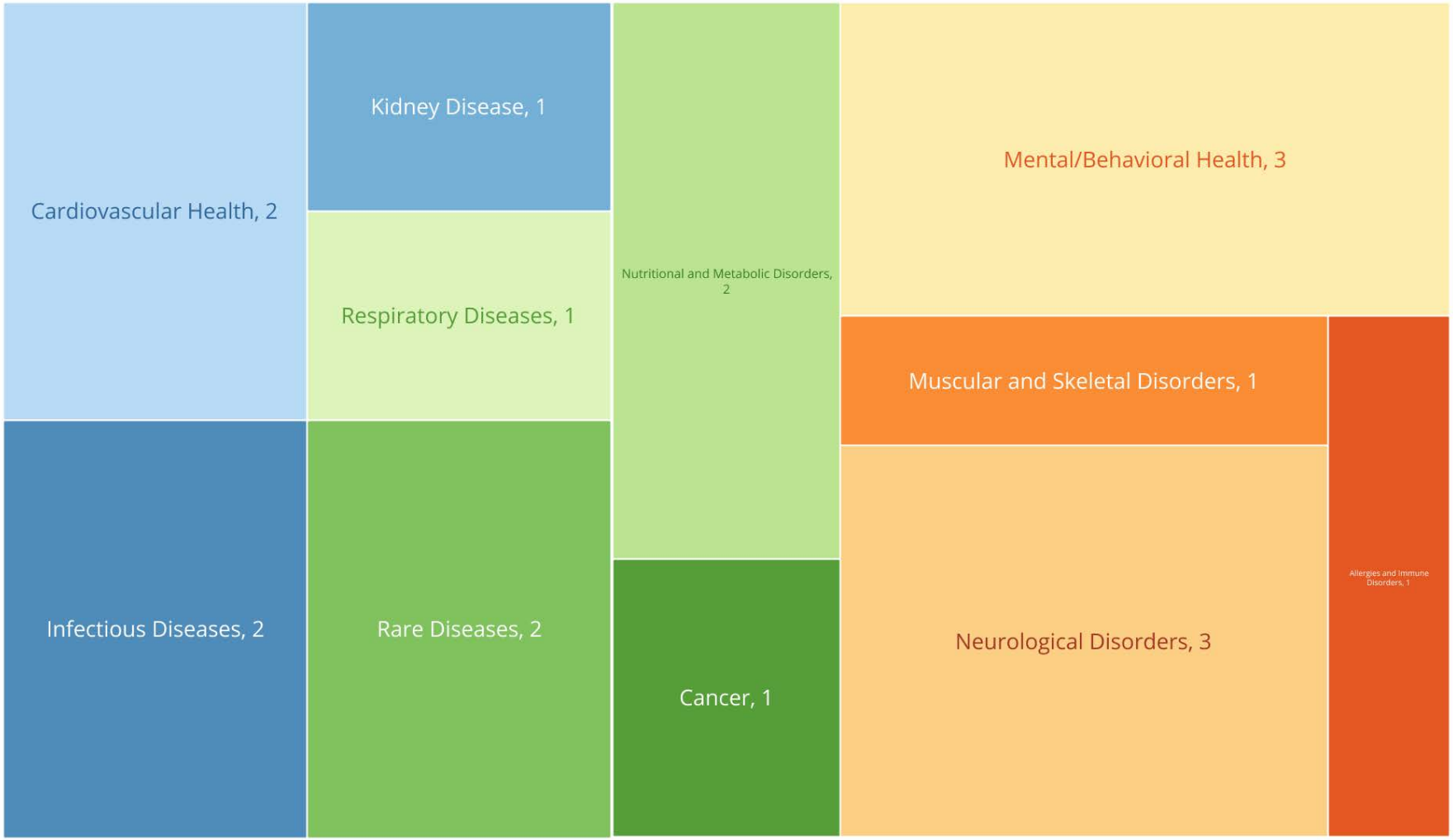
tool not specified

fatigue visual analogue scale

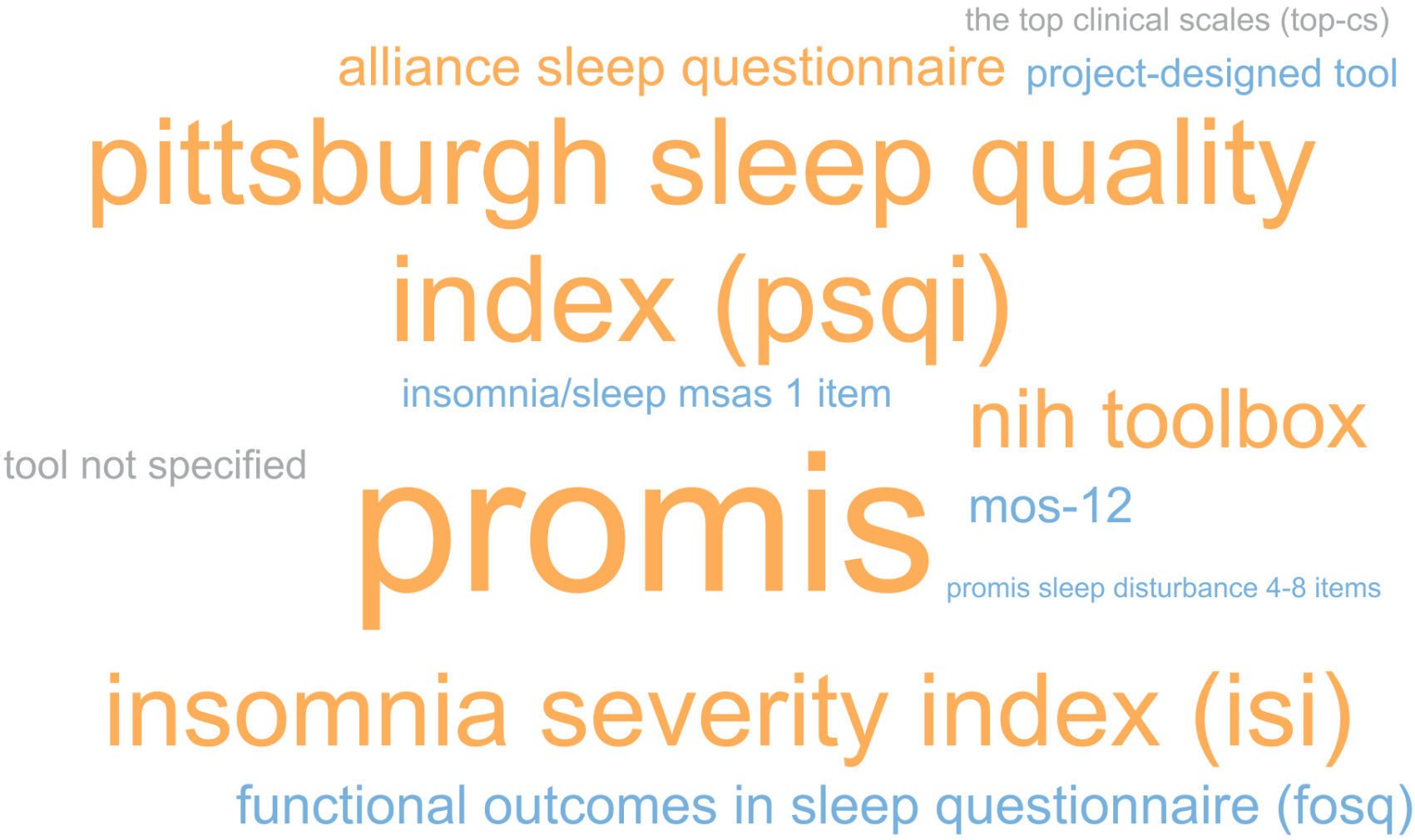
multidimensional fatigue scale

Conditions with Sleep as an Outcome (N=19)

The size of the rectangles correspond to the number of projects with these conditions.



Sleep Measures



Conclusions

- Patient-important outcomes are commonly measured in PCORI's portfolio and across different types of conditions or disease categories
- Measurement approaches are inconsistent, limiting the synthesis of findings across topics
- For some outcomes (pain), the most common tool was either not specified or project defined
- Judicious use of core outcome sets could improve the coherence of PCORI's research portfolio, and even its uptake, if focused on measures applicable to clinical practice

Public Comment Period

Sue Sheridan, MBA, MIM

Director, Patient Engagement

Wrap Up and Adjournment

Grayson Norquist, MD, MSPH
Chairperson, Board of Directors