Board of Governors Meeting

May 04, 2015

10:15 a.m.- 5:45 p.m. ET

PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

Welcome and Introductions

Grayson Norquist, MD, MSPH
Chair, Board of Governors

Joe Selby, MD, MPH
Executive Director

Agenda

Time	Agenda Item
10:15-10:20	Welcome, Call to Order, and Roll Call Consideration of April 21, 2015 Board Meeting Minutes for Approval
10:20-11:10	Executive Director's Report and Mid-year Dashboard Review
11:10-11:40	Mid-year Budget Review
11:40-12:00	**Consider for Approval:** Scientific Publications Committee Charter
12:00-12:15	Public Comment
12:15-1:15	Break
1:15-2:15	**Consider for Approval:** PCORnet Projects
2:15-3:15	Evaluation Update
3:15-3:30	Break
3:30-4:00	Methodology Committee Update
4:00-4:45	Communication and Dissemination Research Program Overview
4:45-5:30	Topic Prioritization Process
5:30-5:45	Public Comment
5:45	Wrap Up and Adjournment

Executive Director's Report

Joe Selby, MD, MPH
Executive Director

Transition

Robin Newhouse, PhD, RN
Chair, Organizational Systems and and Adult Health, University of Maryland School of Nursing
Chair, PCORI MC

Robin Newhouse, PhD, RN
Dean, Indiana University School of Nursing

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Chair, Organizational Systems and and Adult Health, University of Maryland School of Nursing
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Dissemination: Continuing Education for Clinicians

- Focus: Increase awareness of PCORI research findings and methodology standards through high quality CE and CME
- Our CE/CME providers:
  - Baylor College of Medicine
  - PRIME Education
- Audiences:
  - Physicians
  - Physician assistants (PAs)
  - Registered nurses (RNs) and nurse practitioners (NPs)
  - Pharmacists
  - Allied health professionals
- Initial Educational Activities (anticipated end of May 2015):
  - Study design and PCORI's Methodology Standards (Baylor)
  - Early findings about PCORI-funded research comparing intravenous and oral antibiotic treatment for osteomyelitis in children after they leave the hospital (PRIME Education)
- Access to Educational Activities:
  - Links on PCORI's website
  - Free of charge

Update on Peer-Review and Public Release Process

- PCORI and NIH/NLM staff are finalizing technical and other issues to implement the process approved by the Board in February
- RFP for peer review management contractor to be awarded in June
- RFP for contractor to manage lay summaries to be released soon
- Plan in process to modify existing research contracts to include more details on requirements for peer review and public release
- Future PFAs and award contracts will include detailed peer review requirements from the onset
- First funded projects to be peer-reviewed and results released by end of CY 2015

PCORI's First Annual Meeting

Theme: "Progress in Building a Patient-Centered Comparative Effectiveness Research Community"
Date and location: October 6-8, 2015, suburban Washington, DC

Goals:

- Report to the nation on PCORI's achievements and plans for the future
- Highlight PCORI's contributions to CER/PCOR as we mark our fifth anniversary
- General reflection on state of CER/PCOR

Audience:

- Awardees/prospective awardees (researchers and patient/other stakeholder partners)
- Broader stakeholder community interested in PCOR and results of PCORI-funded research, especially Washington, DC-based policy/advocacy community
- Projected attendance: 500-700 people

PCORI's First Annual Meeting

Plenary Sessions:

- Joint session with AHRQ/AcademyHealth featuring multiple stakeholders to showcase dissemination and implementation efforts (Day 1)
- Opening plenary/stakeholder panel on the State of PCOR/CER; Midday plenary on patient engagement (Day 2)

Breakout Sessions (Day 2)
Results from Pilot Projects and other completed projects, organized by program areas/priorities and cross-cutting themes

Learning Labs (Day 2)
"How-to" sessions focused on skill-building and sharing information that will help prospective applicants strengthen proposals for funding

Summits (Day 3)
In-depth "business" meetings for current awardees, organized by major initiative (e.g., PCORnet, Methods, Engagement)

First 10 PCORI Pragmatic Clinical Studies

- **Breast cancer screening tailored to individual risk and preferences vs. annual mammography** for detecting breast cancer and minimizing screening-related harms in women 40-80
- **Annual vs. biennial surveillance CT scanning** in patients found to have small, potentially cancerous growths on initial CT scan
- **Standing order entry system for guiding use of colony stimulating factor vs. usual oncology practice** for reducing over- and underuse of this medication and preventing complications in patients with breast, lung, colorectal cancer
- **Comprehensive transitional care program of early discharge and in-home support services vs. usual care** in improving functional status and preventing hospital readmissions and mortality in stroke survivors
- **Primary care plus prompt referral to physical therapy and cognitive behavioral therapy vs. usual primary care** to prevent acute back pain from becoming chronic

First 10 PCORI Pragmatic Clinical Studies

- **Healthy lifestyle intervention plus metformin therapy vs. health lifestyle intervention** alone for reducing weight gain and metabolic problems associated with certain antipsychotic medications in youth with bipolar disorders
- **Anti-TNF factor vs. anti-TNF plus low dose of methotrexate** in children with Crohn's disease for induction, maintenance of remission, patient-reported outcomes, and adverse events
- **Nerve blocking regional anesthesia vs general anesthesia** in older adults undergoing surgery for hip fracture on acute post-operative pain, satisfaction with care, inpatient morbidity, and ability walk without assistance at 60 and 180 days, health and disability, pain, ability to return home after fracture, and mortality
- **Exercise coaching program vs. usual care** for older adults who have experienced a low-impact fracture as a result of a fall for preventing further injuries and improving health
- **Proton-beam vs. photon-beam radiation therapy** post-mastectomy in women with Stage II or III for outcomes of recurrence, mortality and cardiovascular disease complications of radiation therapy

Current evaluation activities related to Pragmatic Clinical Studies

PCORI allocated enough to fund 6-9 applications in each cycle and awarded 5 per cycle. To increase the number of awards in subsequent cycles, we are:

- Actively reaching out to research community to increases awareness and increase the numbers of LOIs and applications for PCS awards
- Working with stakeholder organizations to increase the number of high priority topics, many of which will go to the PCS high-priority list
- Refining the RFA to be clearer on requirements/expectations
- Working with merit review teams to assure that reviews appropriately capture PCORI review criteria
- Considering increasing proportion of LOI's invited to submit full proposals
- Anticipating arrival of resubmitted LOI's and applications which should augment the numbers of high-scoring applications
- Evaluating researcher readiness to organize and lead pragmatic studies
- Surveying researchers who have recently published good CER to assess their awareness of and attitudes toward PCORI as a funding source

Review of Today's Agenda

- FY 2015 Mid-year Dashboard
- FY 2015 Mid-year Financial Update
- Scientific Publications Committee Charter - for approval
- PCORnet Demonstration Projects - for approval
- Methodology Committee Update
- Evaluation Update
- Update/Overview - PCORI's Communication and Dissemination Program
- PCORI's Topic Prioritization Process

Questions

- Does the Board have other suggestions for increasing the number of high-quality pragmatic clinical studies applications and subsequent awards?
- Comments/questions on our Annual Meeting plans?

Dashboard Review
Mid-year FY 2015

Joe Selby, MD, MPH
Executive Director

Michele Orza, ScD
Senior Advisor to the Executive Director

Presentation Overview

- Q2 FY 2015 Dashboard
- Noteworthy Items
- Yellow-flagged Items

However beautiful the strategy,

you should occasionally look at the results.

Winston Churchill

Discussion Questions

- Do you see the ***need for further action*** in response to any of the indicators discussed today?
- What ***further improvements*** should we consider so that our Dashboard and accompanying materials ***effectively convey the status of our work and progress toward our goals***?

pcori

Board of Governors FY2015 Dashboard – Q2 (As of 3/31/2015)

Influencing Research

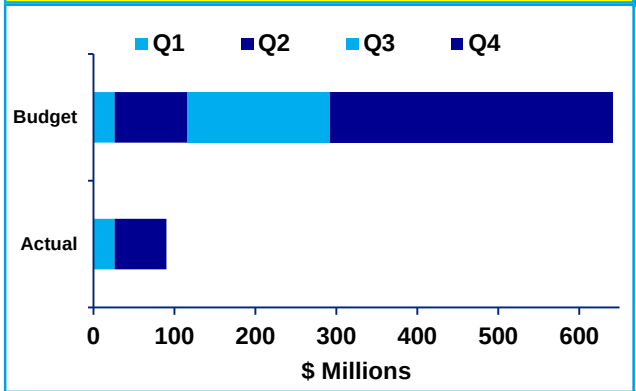
The University of Pittsburgh credits PCORI with being the inspiration for and central to the establishment of their Comparative Effectiveness Research Core

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

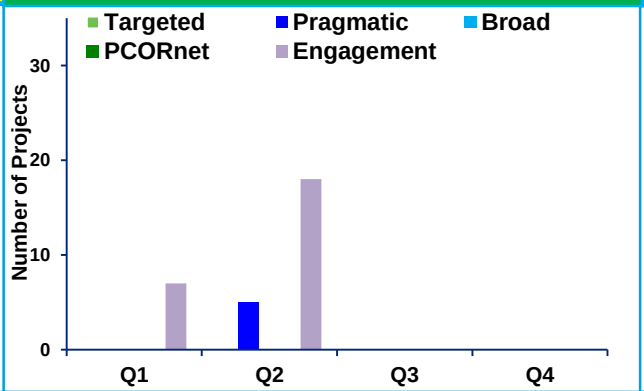
Legend

- On Target
- Off Target
- Needs Attention
- NA=Not Applicable
- Q3=Q3 2014
- Q4=Q4 2014
- Q1=Q1 2015
- Q2=Q2 2015

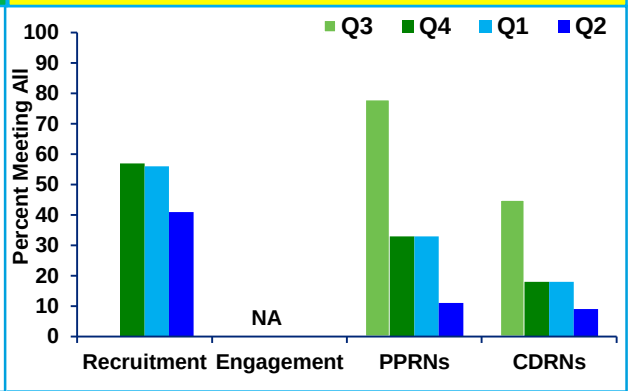
Funds Committed to Research – Budget=$640M



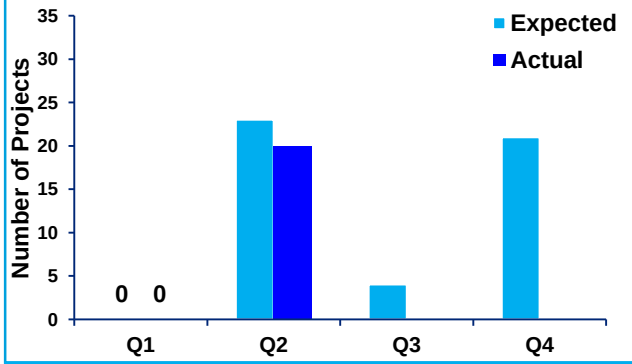
Projects Awarded



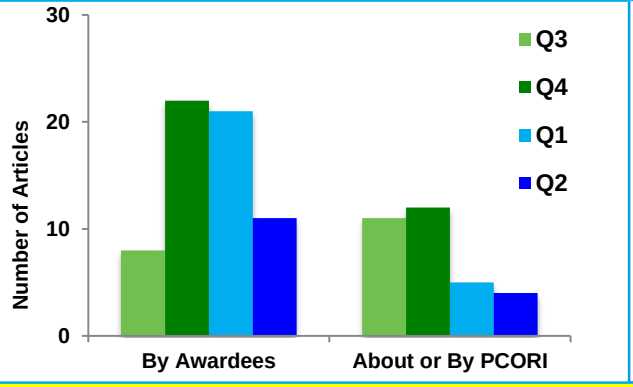
Percent of Projects Meeting All Milestones



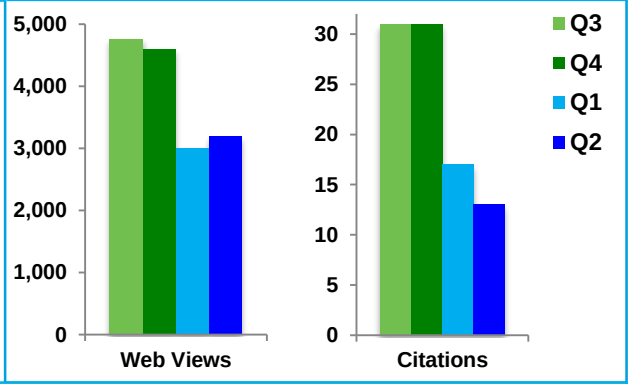
Completion of Projects



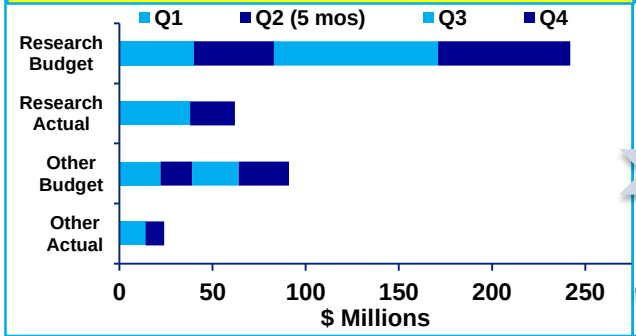
Journal Articles Published



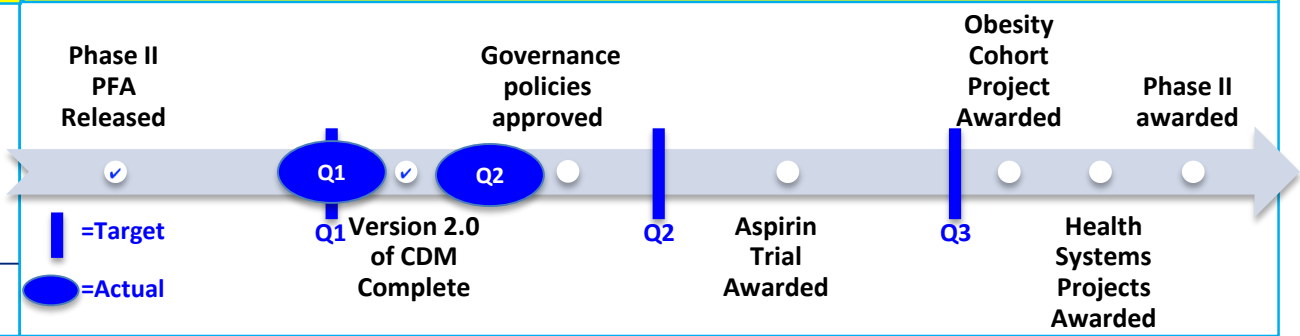
Uptake of Methodology Standards



Expenditures – Total Budget=$362M



Progress of PCORnet – Completion of Phase I



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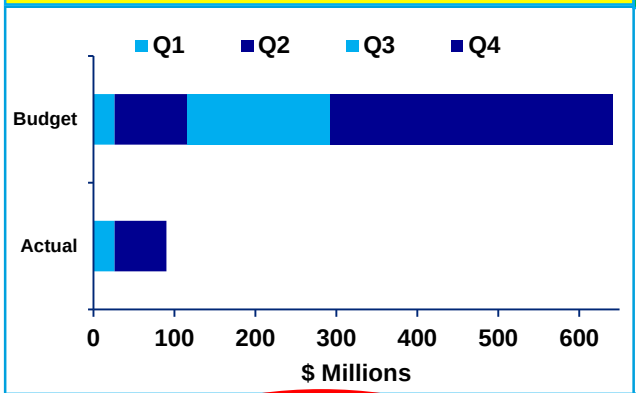

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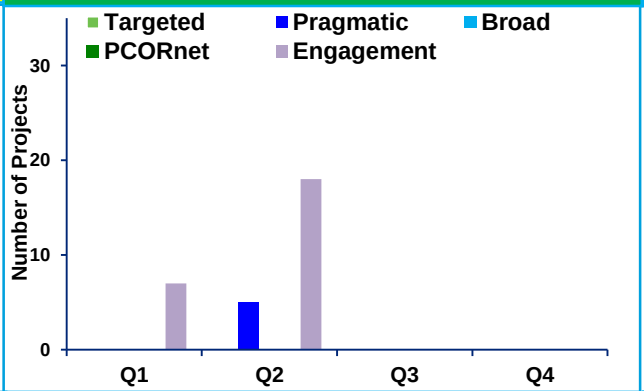

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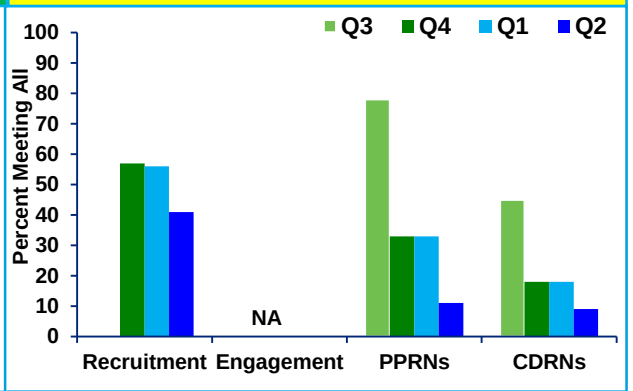

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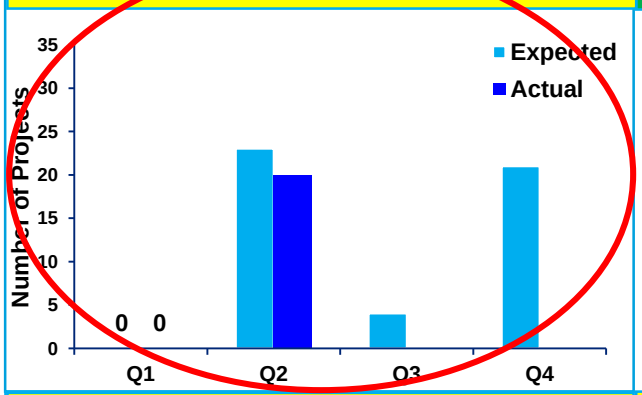

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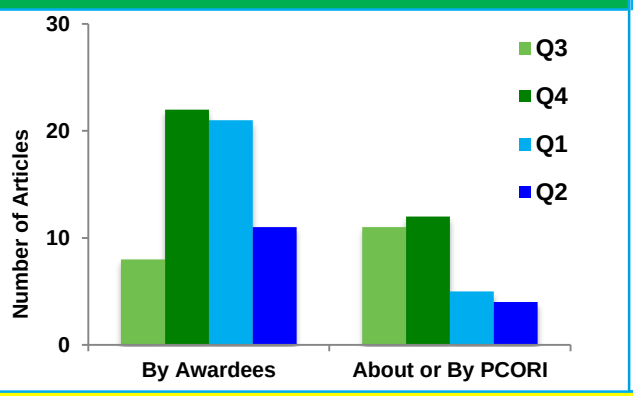

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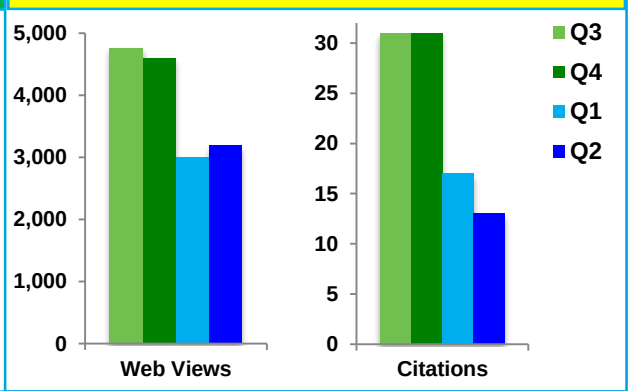

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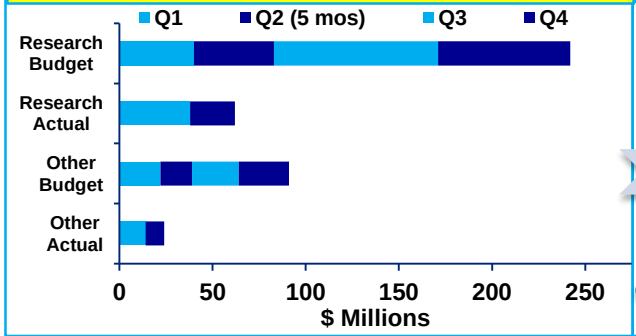

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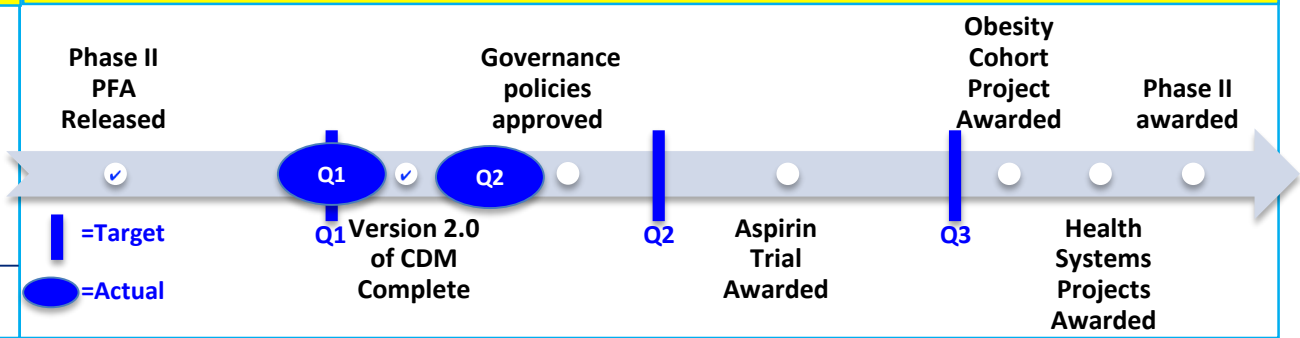

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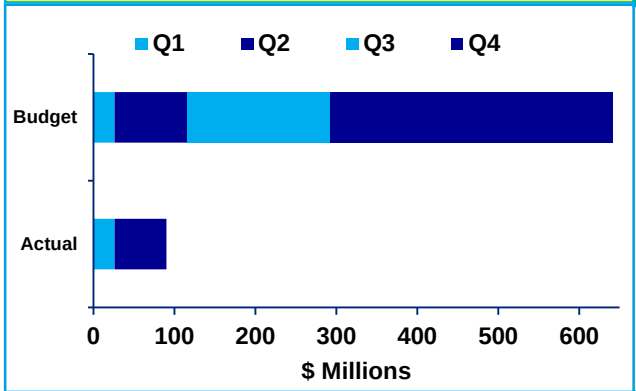

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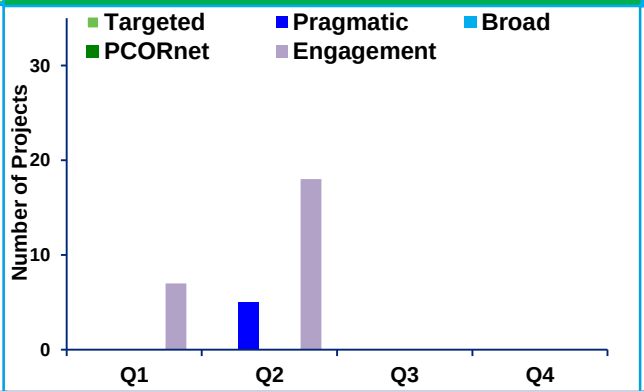

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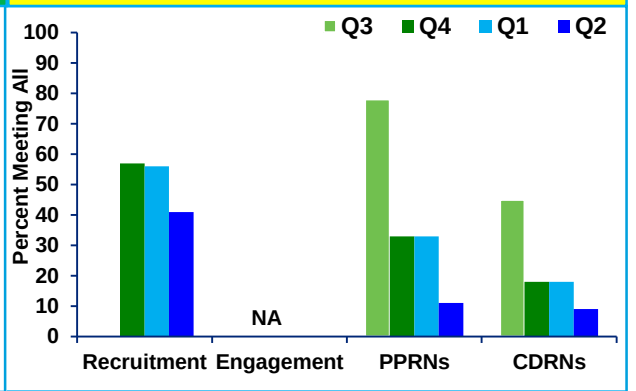

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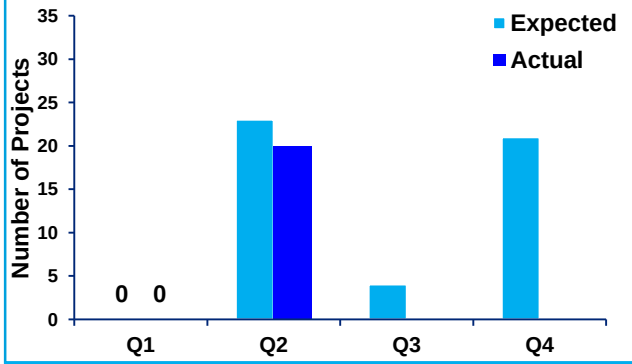

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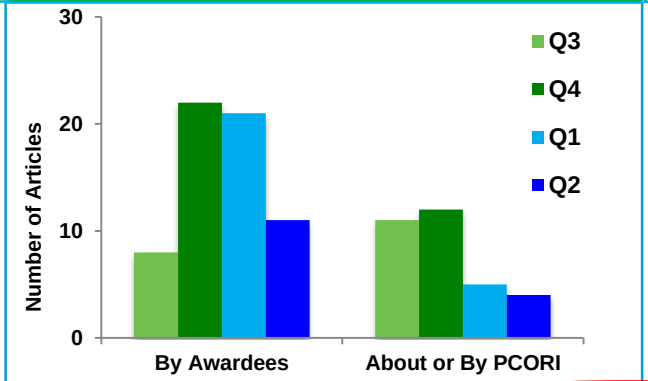

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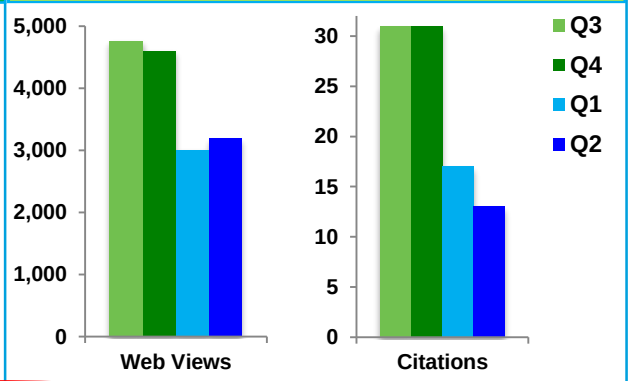

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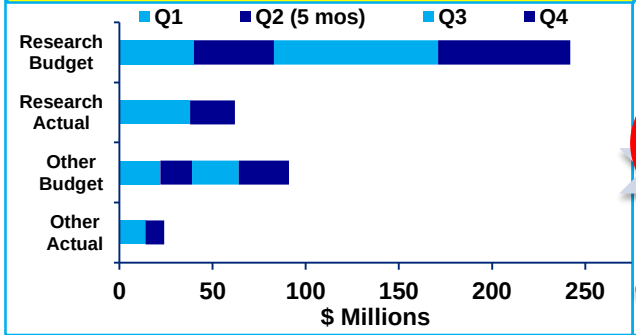

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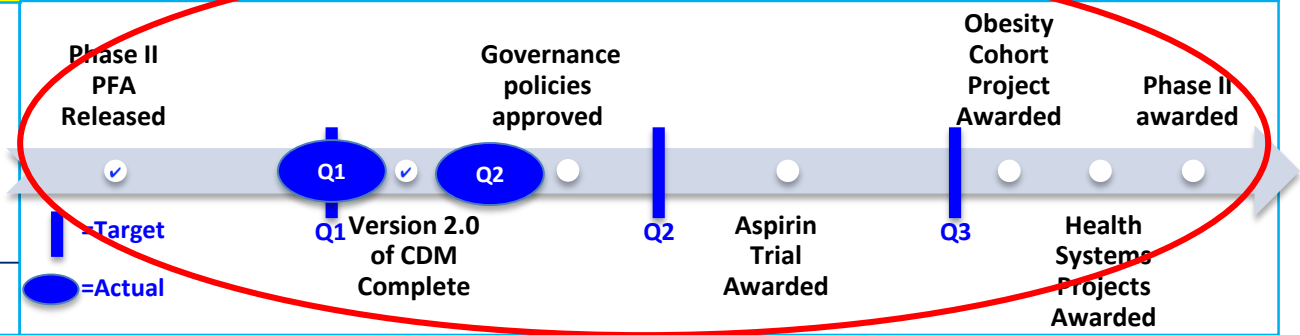


Early Signs of Influence on Research

Influencing Research

The University of Pittsburgh credits PCORI with being an inspiration for and central to the establishment of their Comparative Effectiveness Research Center

The Comparative Effectiveness Research Center (CERC) at the University Of Pittsburgh and UPMC

- Established in 2011 to support Patient-Centered CER at the University of Pittsburgh and UPMC
- Interest in developing this infrastructure stemmed from
  - desire to promote collaborative PC-CER across the University and UPMC
  - availability of new funding sources, such as PCORI
- CERC aims to:
  - Support high-quality PC-CER across the University through infrastructure support, training, collaborations, and strategic coordination of responses to funding opportunities
  - Promote the University's PC-CER externally to increase funding opportunities
  - Develop new statistical and methodological approaches to advance the science of PC-CER
  - Expand the pool of researchers trained in PC-CER via interactive workshops, seminars, and meetings
  - Demonstrate the translation of PC-CER via dissemination and implementation into actions that effectively reach the patients and directly impact clinical care

"PCORI is central to the CERC and has greatly influenced work across the University"

Sally Morton, Director of CERC and PCORI Methodology Committee Member

Early Signs of Influence on Research

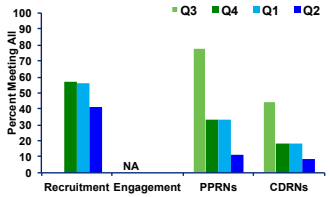
Influencing Research

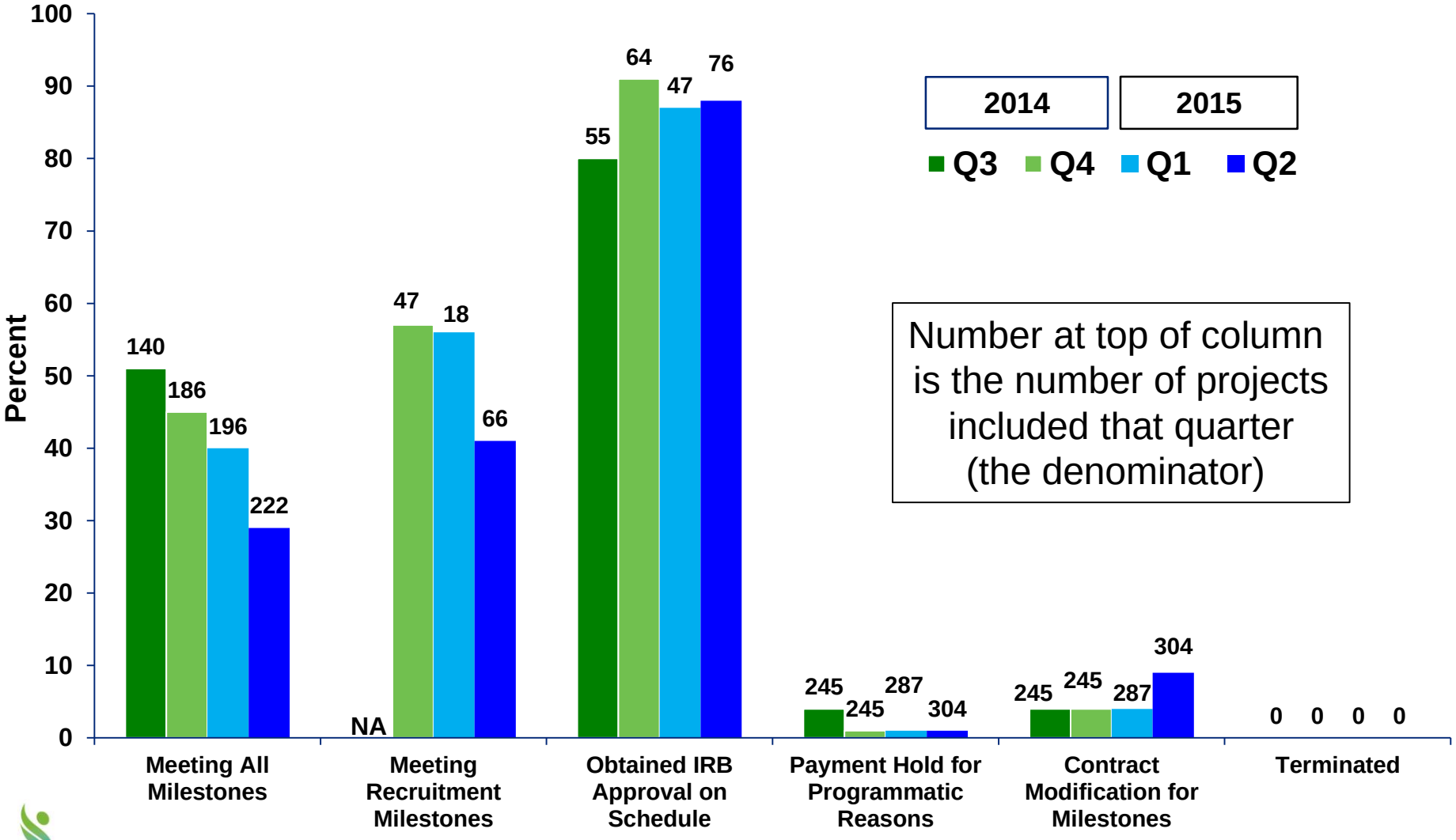
The University of Pittsburgh credits PCORI with being an inspiration for and central to the establishment of their Comparative Effectiveness Research Center

At the University of Pittsburgh, PCORI is credited with motivating their:

- Establishment of a HIPAA compliant **data center:**
  - 20 projects currently using it
  - $13 million across all projects (PCORI and other funders)
- Development of **training and educational opportunities:**
  - Graduate courses and training grants (AHRQ-funded) based on the PCORI Methodology Standards
  - 54 training workshops since 2011 on PC-CER funding opportunities and review criteria, PC-CER methodology, and stakeholder engagement
  - Mock reviews for PCORI applications (assess engagement, adherence to standards)
- Emphasis on **stakeholder engagement:**
  - Influence apparent in existing projects
  - *"These are new concepts for some of our researchers – PCORI is making them think about the stakeholders and how they can qualify to be a PCORI project"*
    – Monica Costlow, CERC Project Director
- Encouragement of people at the University and UPMC to apply to be **PCORI reviewers** and to get involved in other PCORI activities

Progress of Research Projects: Additional Measures





Articles Resulting from Funded Projects – Q2

- J Sussman, D Kent et al. **Improving Diabetes Prevention with Benefit Based Tailored Treatment: Risk Based Reanalysis of Diabetes Prevention Program**. *BMJ* February 2015 (Impact factor 16.3) – 2012 Pilot Project award
- H Angier et al. **An Early Look at Rates of Uninsured Safety Net Clinic Visits after the Affordable Care Act**. *Annals of Family Medicine* January/February 2015 (Impact factor 4.6) – 2012 IHS award
- R Keren et al. **Comparative Effectiveness of Intravenous vs Oral Antibiotics for Postdischarge Treatment of Acute Osteomyelitis in Children**. *JAMA Pediatrics* February 2015 (Impact factor 4.3) – 2012 ADPTO award. Related editorial by D Pranita et al.
- T Lieu, G Thomas Ray et al. **Geographic Clusters in Underimmunization and Vaccine Refusal**. *Pediatrics* February 2015 (Impact factor 5.3) – 2012 Pilot Project award
- M Gilman, EK Adams et al. **Safety-Net Hospitals More Likely Than Other Hospitals to Fare Poorly Under Medicare's Value-Based Purchasing**. *Health Affairs* March 2015 (Impact factor 4.3) – 2012 Pilot Project award

Articles Resulting from Funded Projects – Q2

- M VonKorff, R Palmer et al. **The Prevalence of Problem Opioid Use in Patients Receiving Chronic Opioid Therapy: Computer Assisted Review of Electronic Health Record Clinical Notes**. *Pain* March 2015 (Impact factor 4.1) – 2013 IHS award
- M Kahn, J Brown et al. **Transparent Reporting of Data Quality in Distributed Data Networks**. *eGEMs* March 2015 – 2013 Methods award.
- A Porter, D Hynes et al. **Rationale and Design of a Patient-Centered Medical Home Intervention for Patients with End-Stage Renal Disease on Hemodialysis**. *Contemporary Clinical Trials February 2015 (Impact factor 1.9) – 2013 IHS award*
- J Eyer and B Thorn. **The Learning About My Pain Study Protocol: Reducing Disparities with Literacy-Adapted Psychosocial Treatments for Chronic Pain**. *Journal of Health Psychiatry* February 2015 (Impact factor 1.8) – 2012 AD award
- S Mikles and T Mielenz. **Characteristics of Electronic Patient-Provider Messaging Utilization in Urban Health Care Organization**. *Journal of Innovation in Health Informatics* January 2015 – 2012 Pilot Project award
- H Witteman, S Dansokho et al. **User-centered design and the development of patient decision aids: protocol for a systematic review**. Systematic Reviews January 2015 – 2013 Methods award

PCORI in the Literature

This collection of articles and opinion pieces provides insights into PCORI's work to advance patient-centered comparative clinical effectiveness research. The collection includes papers, articles, and commentaries written by researchers working on PCORI-funded projects and by PCORI staff, members of our Board of Governors and Methodology Committee, and others who are following our work.

PCORI is committed to the principle of transparency and openness in all of our work, including the sharing of information about our activities and the dissemination of research findings. We encourage authors to make their articles available without a subscription. In the list below, we provide links when articles are available without charge.

Results from PCORI-Funded Research Studies

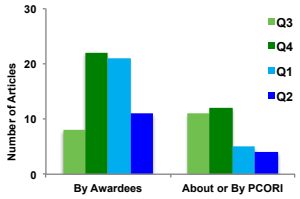
An Early Look at Rates of Uninsured Safety Net Clinic Visits After the Affordable Care Act
Heather Angier, Megan Hoopes et al., "An Early Look at Rates of Uninsured Safety Net Clinic Visits After the Affordable Care Act," *Annals of Family Medicine* 13(1) (January/February 2015): 10-16.
View PCORI Study

Improving Diabetes Prevention with Benefit Based Tailored Treatment: Risk Based Reanalysis of Diabetes Prevention Program
Jeremy B. Sussman, David M. Kent, Jason P. Nelson, and Rodney A. Hayward, "Improving Diabetes Prevention with Benefit Based Tailored Treatment: Risk Based Reanalysis of Diabetes Prevention Program," *BMJ* 350 (February 2015): h454.
View PCORI Study

Advance Care Planning: A Qualitative Study of Dialysis Patients and Families Abstract only available
Sarah L. Goff, Nwamaka D. Eneanya et al., "Advance Care Planning: A Qualitative Study of Dialysis Patients and Families," *Clinical Journal of the American Society of Nephrology* (February 2015) Published online DOI: 10.2215/CJN.07490714.
View PCORI Study

Rationale and Design of a Patient-Centered Medical Home Intervention for Patients with End-Stage Renal Disease on Hemodialysis Abstract only available
Anna C. Porter, Denise M. Hynes et al., "Rationale and Design of a Patient-Centered Medical Home Intervention for Patients with End-Stage Renal Disease on Hemodialysis," *Contemporary Clinical Trials* 42 (February 2015): 1-8.
View PCORI Study

Additional Metrics for Early Dissemination and Uptake



Starting in Q3

- Average Impact Factor
- Percent of Articles in Top Tier Journals

Starting in Q4

- Citations
- Alternative Metrics (such as media coverage)
- Uptake (such as into systematic reviews or guidelines)

Discussion Questions

- Do you see the ***need for further action*** in response to any of the indicators discussed today?
- What ***further improvements*** should we consider so that our Dashboard and accompanying materials ***effectively convey the status of our work and progress toward our goals***?

pcori

Board of Governors FY2015 Dashboard – Q2 (As of 3/31/2015)

Influencing Research

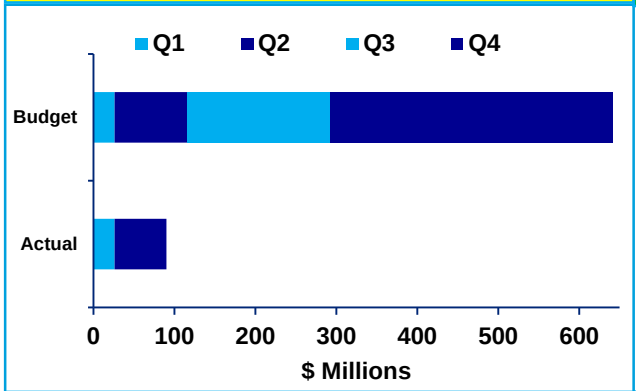
The University of Pittsburgh credits PCORI with being the inspiration for and central to the establishment of their Comparative Effectiveness Research Core

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

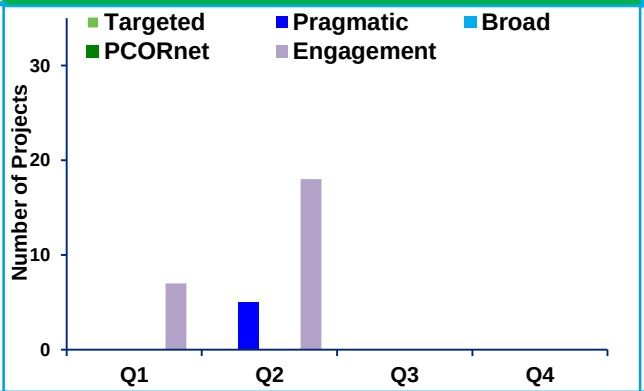
Legend

- On Target
- Off Target
- Needs Attention
- NA=Not Applicable
- Q3=Q3 2014
- Q4=Q4 2014
- Q1=Q1 2015
- Q2=Q2 2015

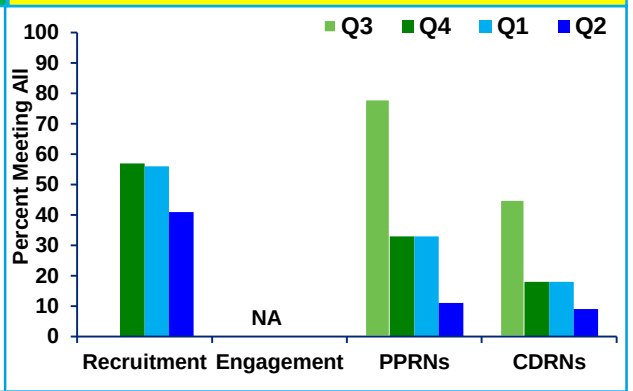
Funds Committed to Research – Budget=$640M



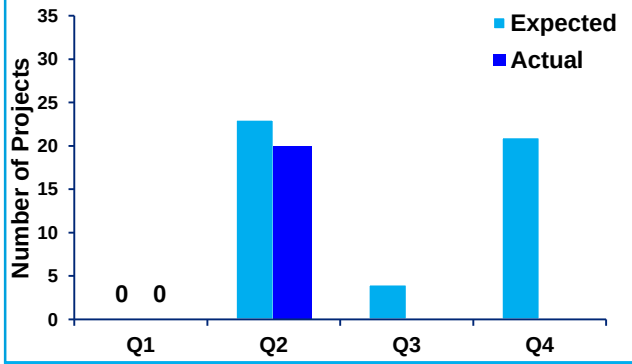
Projects Awarded



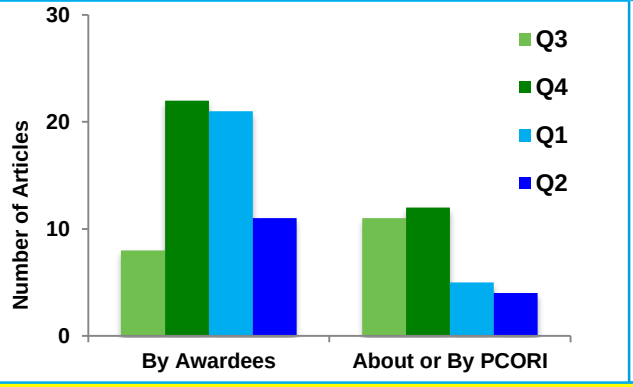
Percent of Projects Meeting All Milestones



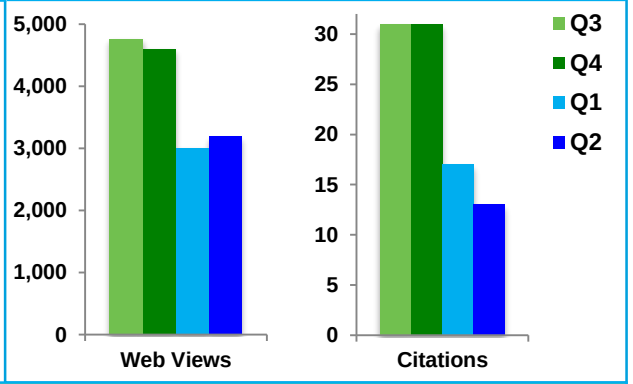
Completion of Projects



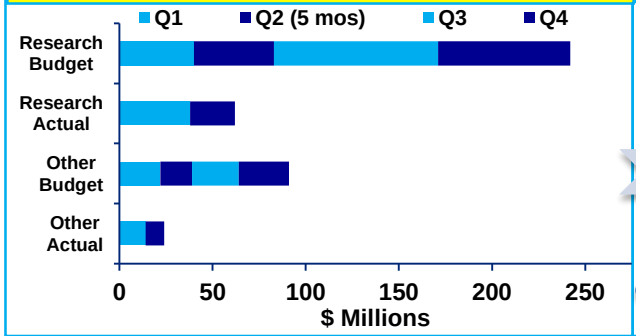
Journal Articles Published



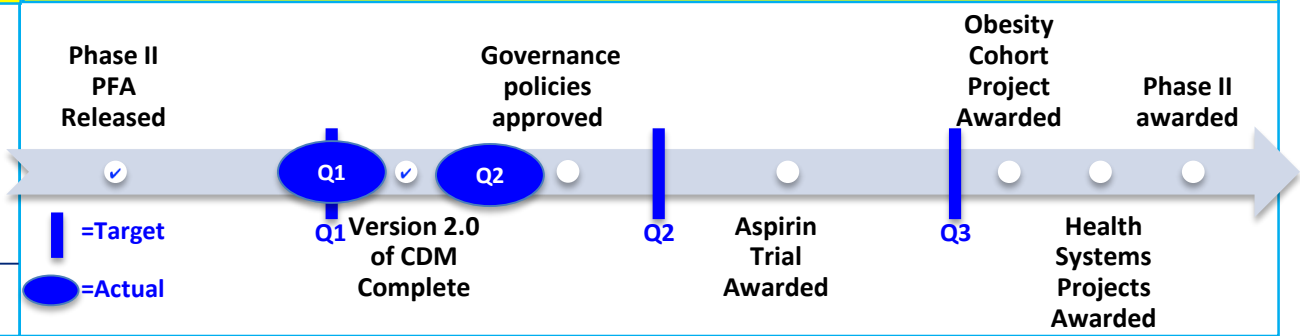
Uptake of Methodology Standards



Expenditures – Total Budget=$362M



Progress of PCORnet – Completion of Phase I

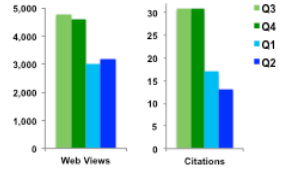


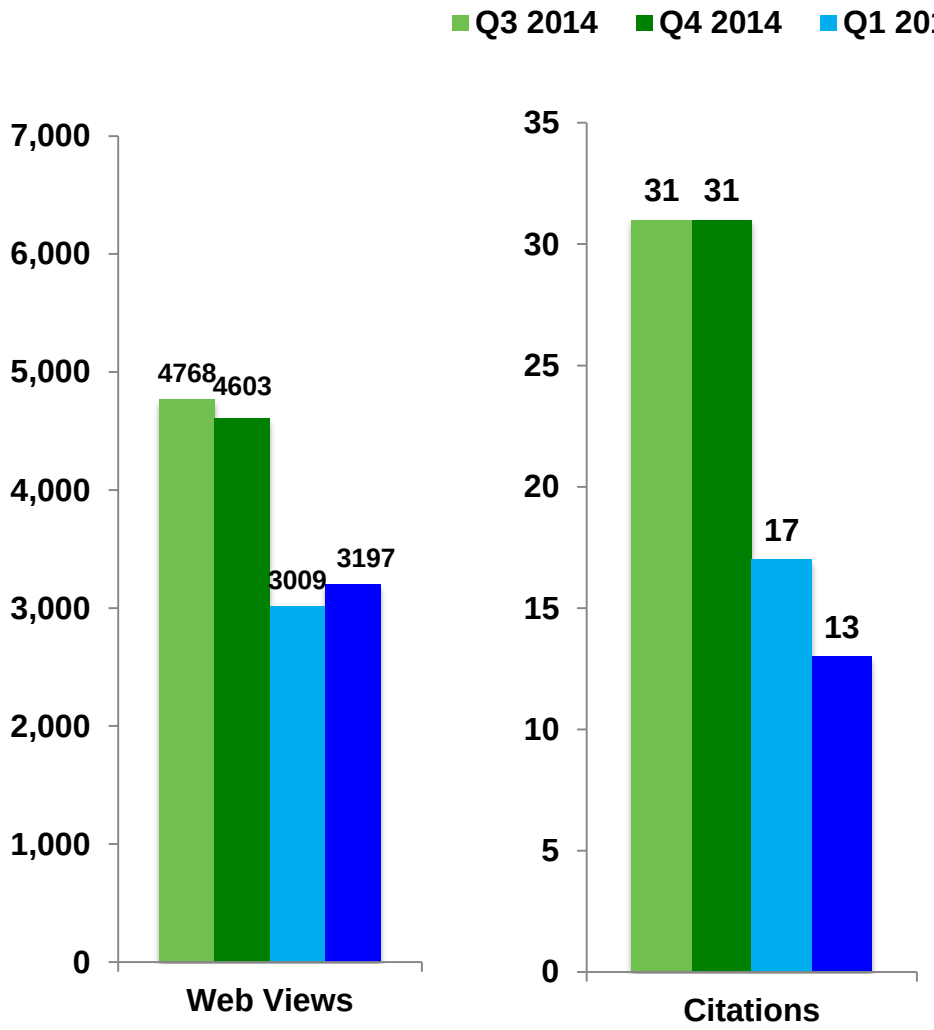
Appendix

Slides available to answer questions about Methodology Standards use and uptake:

- Adherence of Awarded Applications
- Use by Researchers
- Experience of Applicants
- Experience of Merit Reviewers

Uptake of Methodology Standards





We are also tracking:

- Adoption
- Endorsements
- CE/CME (Q3)
- Uptake into Curriculum
- Use of PCORI-developed Curriculum (2016)

Adherence of Awarded CER* Applications to PCORI's Methodology Standards at Time of Award

Adherence by Standard Category (average across 3 cycles - 88 applications)

Category	Standard	Number of projects (N)	% Adherence
Standards for Formulating Research Questions	**RQ-1** Identify Gaps in Evidence	**68**	**80%**
	RQ-3 Identify Specific Populations and Health Decision(s) Affected by the Research	**87**	**98%**
	RQ-4 Identify and Assess Participant Subgroups	**46**	**74%**
	RQ-5 Select Appropriate Interventions and Comparators	**86**	**98%**
	RQ-6 Measure Outcomes that People Representing the Population of Interest Notice and Care About	**87**	**99%**
Patient-Centeredness	**PC-1** Engage people representing the population of interest and other relevant stakeholders in ways that are appropriate and necessary in a given research context.	**85**	**98%**
	PC-2 Identify, Select, Recruit, and Retain Study Participants Representative of the Spectrum of the Population of Interest and Ensure that Data Are Collected Thoroughly and Systematically from All Study Participants	**81**	**93%**
	PC-3 Use Patient-Reported Outcomes When Patients or People at Risk of a Condition Are the Best Source of Information	**80**	**97%**
	PC-4 Support dissemination and implementation of study results	**83**	**95%**

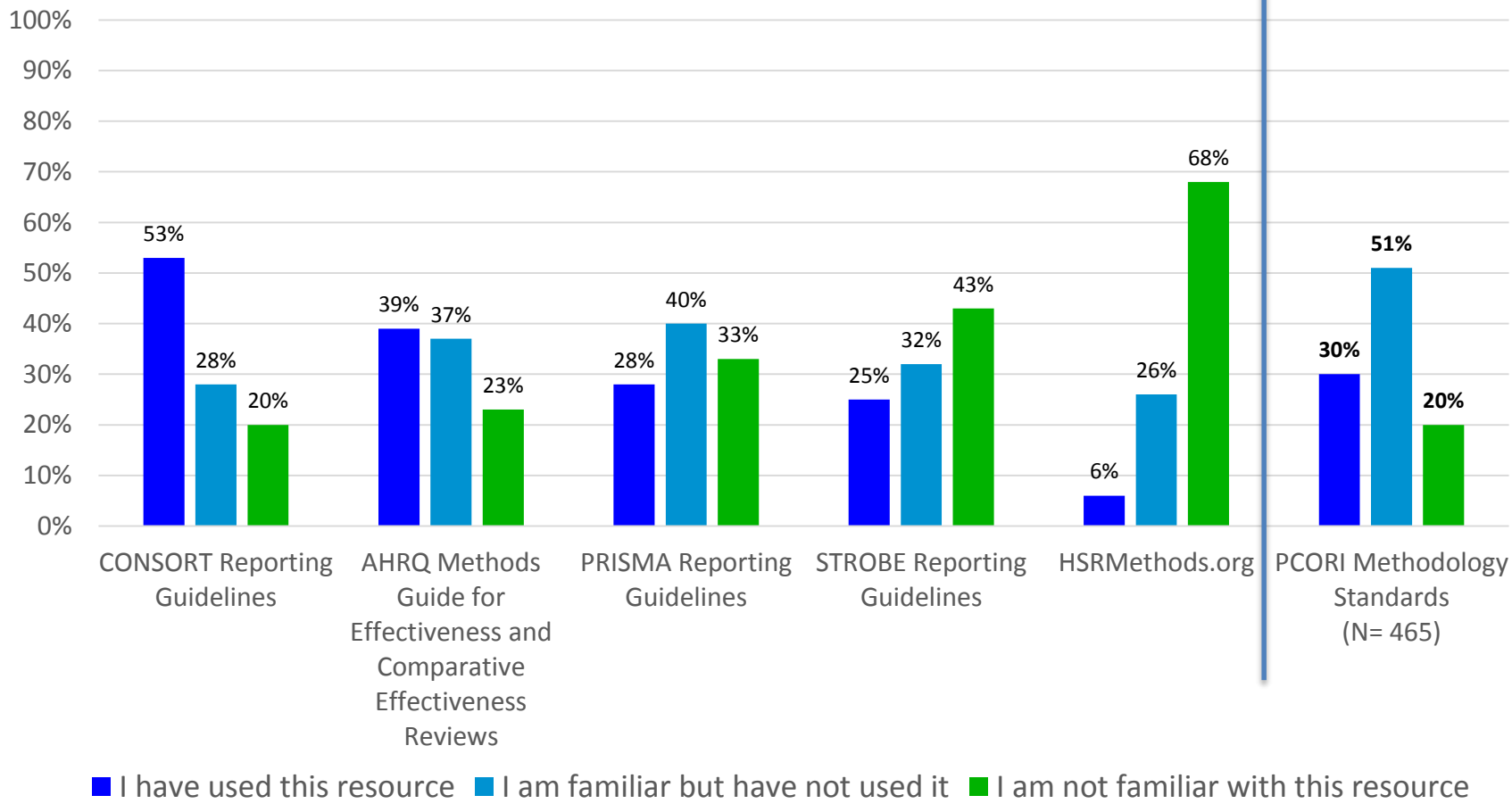
Adherence of Awarded CER* Applications to PCORI's Methodology Standards at Time of Award

Adherence by Standard Category (average across 3 cycles – 88 applications)

	Standard Category	Number of projects (N)	% Adherence
Standards for Data Integrity and Rigorous Analyses	**IR-1** Assess Data Source Adequacy	**41**	**57%**
	IR-2 Describe Data Linkage Plans, if Applicable	**13**	**69%**
	IR-3 A priori, Specify Plans for Data Analysis that Correspond to Major Aims	**80**	**95%**
	IR-4 Document Validated Scales and Tests	**71**	**92%**
Standards for Heterogeneity of Treatment Effect (HTE)	**HTE-1** State the Goals of HTE Analyses	**28**	**65%**

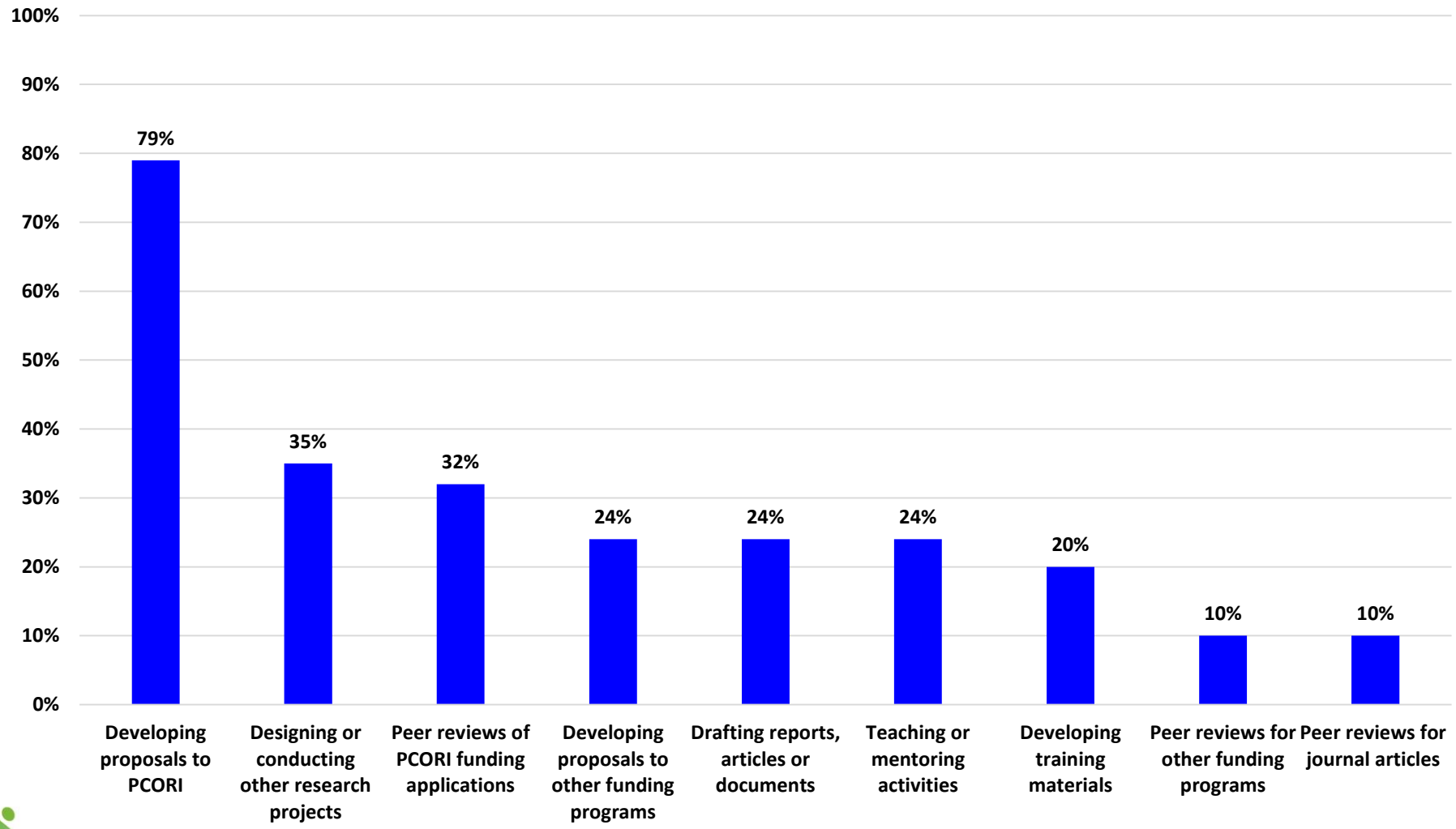
*Does not include Methods applications

From Our Survey of Researchers: Which best describes your experience with the following resources for research methods? (N=506)

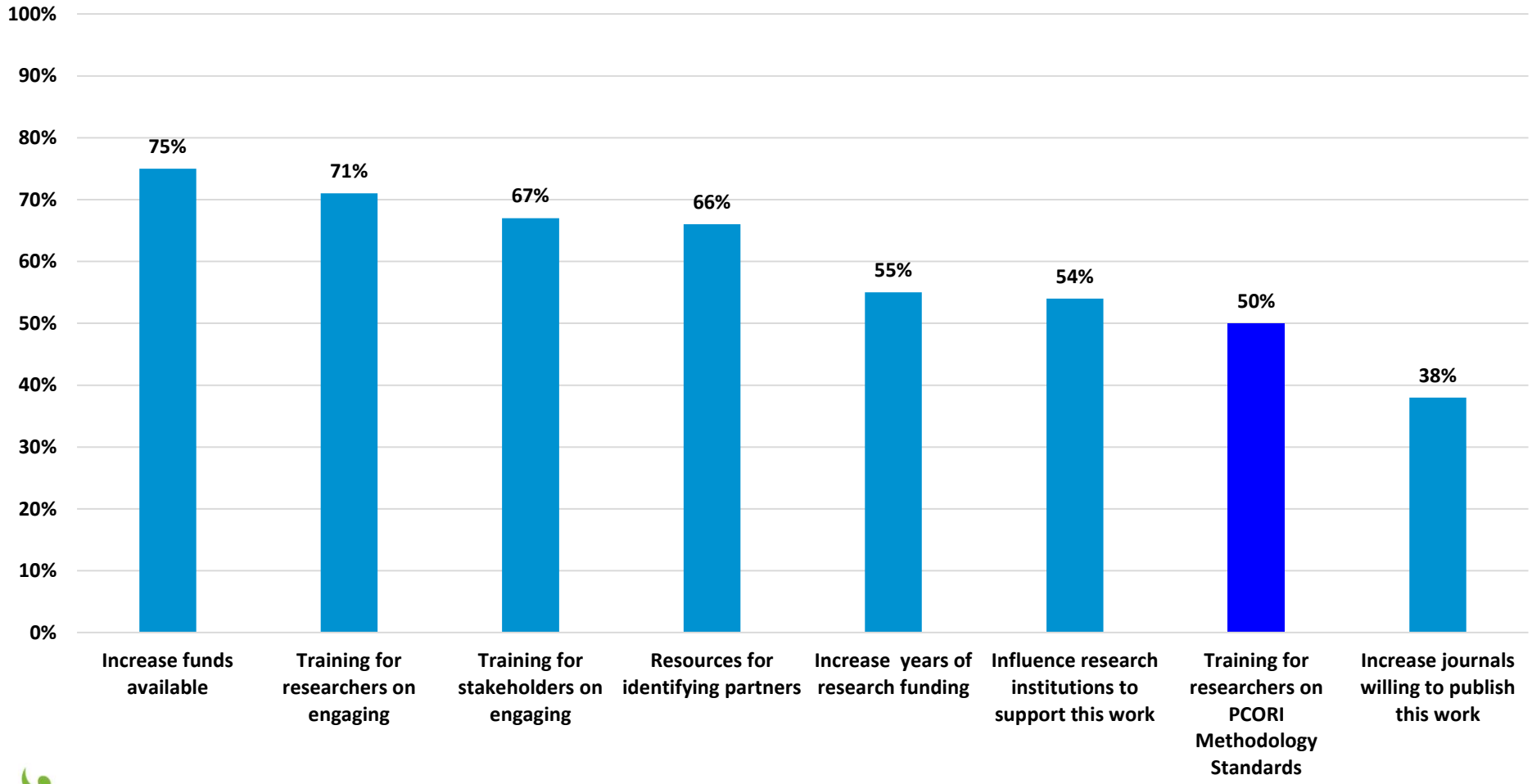


Consolidated Standards of Reporting Trials (CONSORT) reporting guidelines; Preferred Reporting Items for Systematic Reviews and Meta-Analyses (PRISMA) reporting guidelines; Strengthening the Reporting of Observational Studies in Epidemiology (STROBE) reporting guidelines

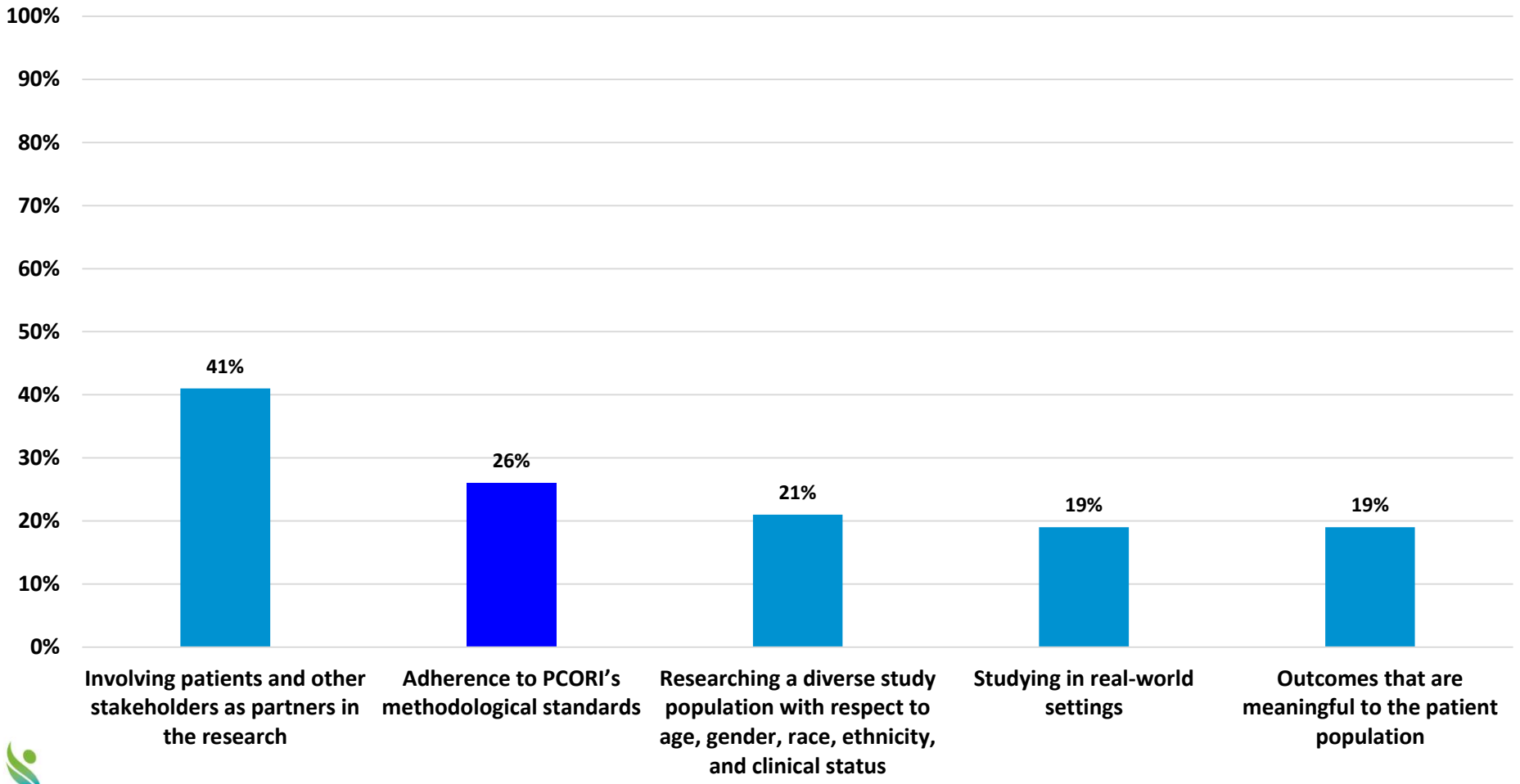
From Our Survey of Researchers: Please indicate the activities for which you used the PCORI Methods Standards. Mark all that apply. (N=135)



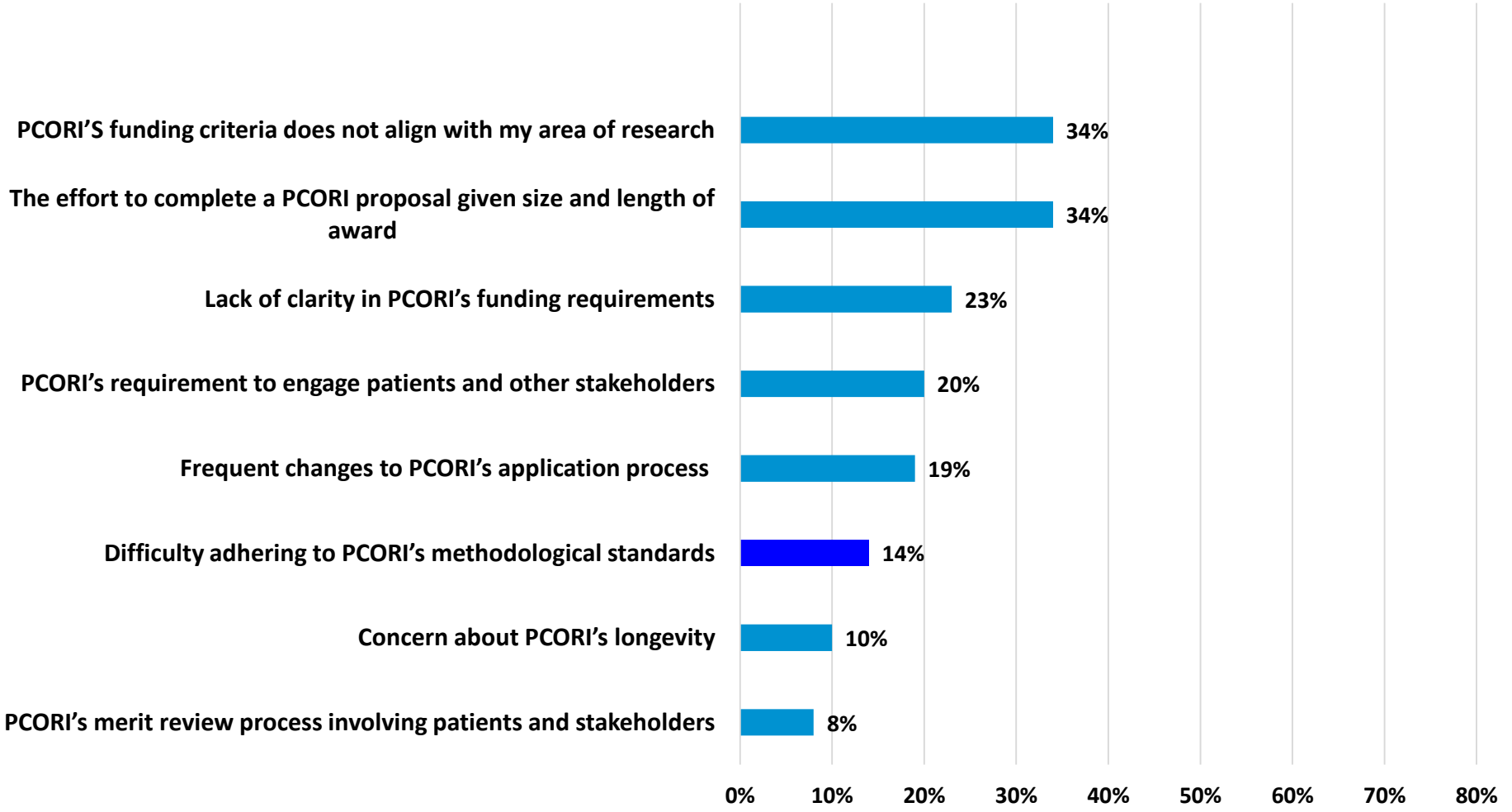
From Our Survey of Researchers: What could be done to encourage researchers to involve patients and/or caregivers as partners? Mark all that apply. (N=465)



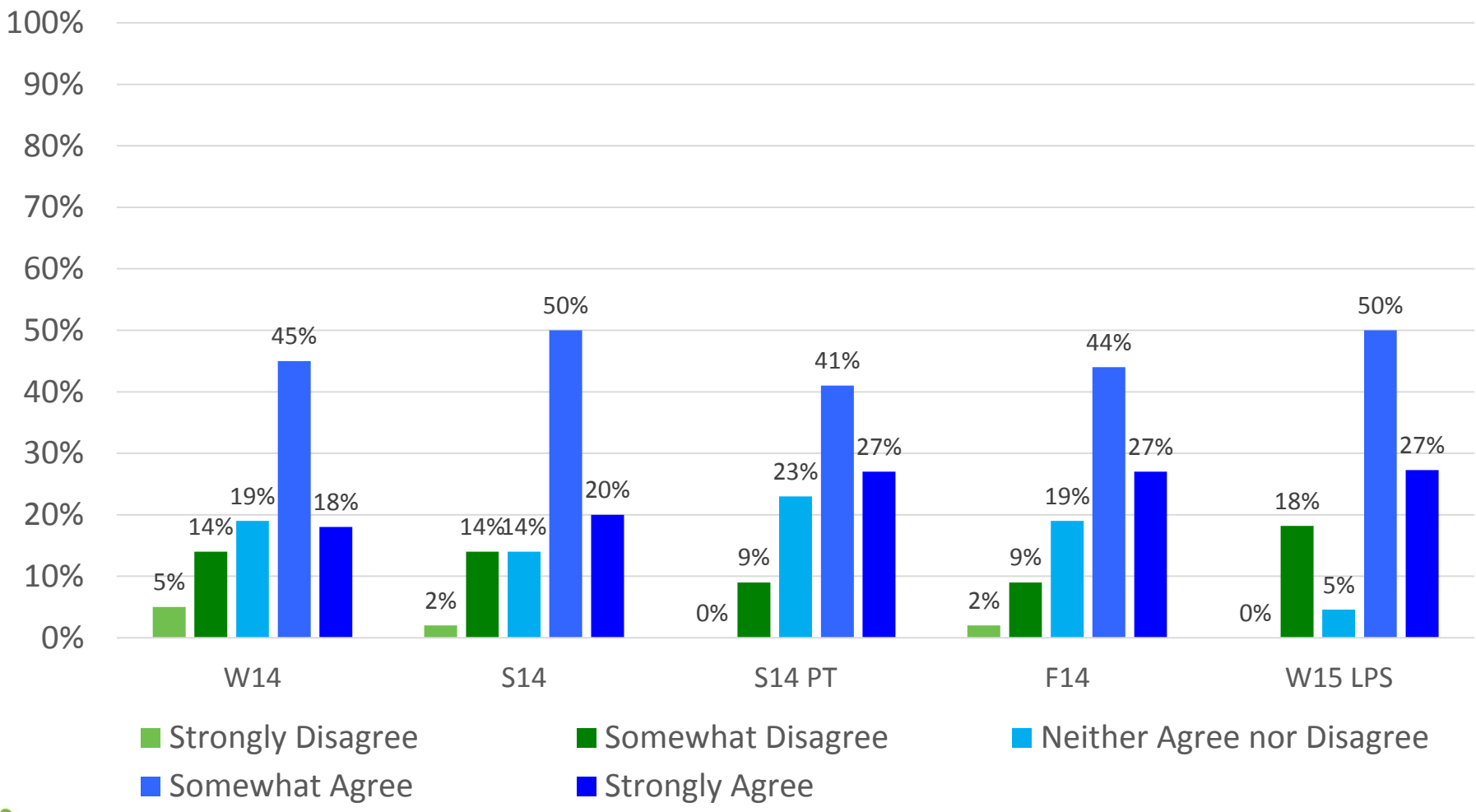
From Our Researcher Survey: How difficult was it for you to respond to the following PCORI application criteria when proposing your study design? (N=272) Percent responding 'Very Difficult' or 'Somewhat Difficult'



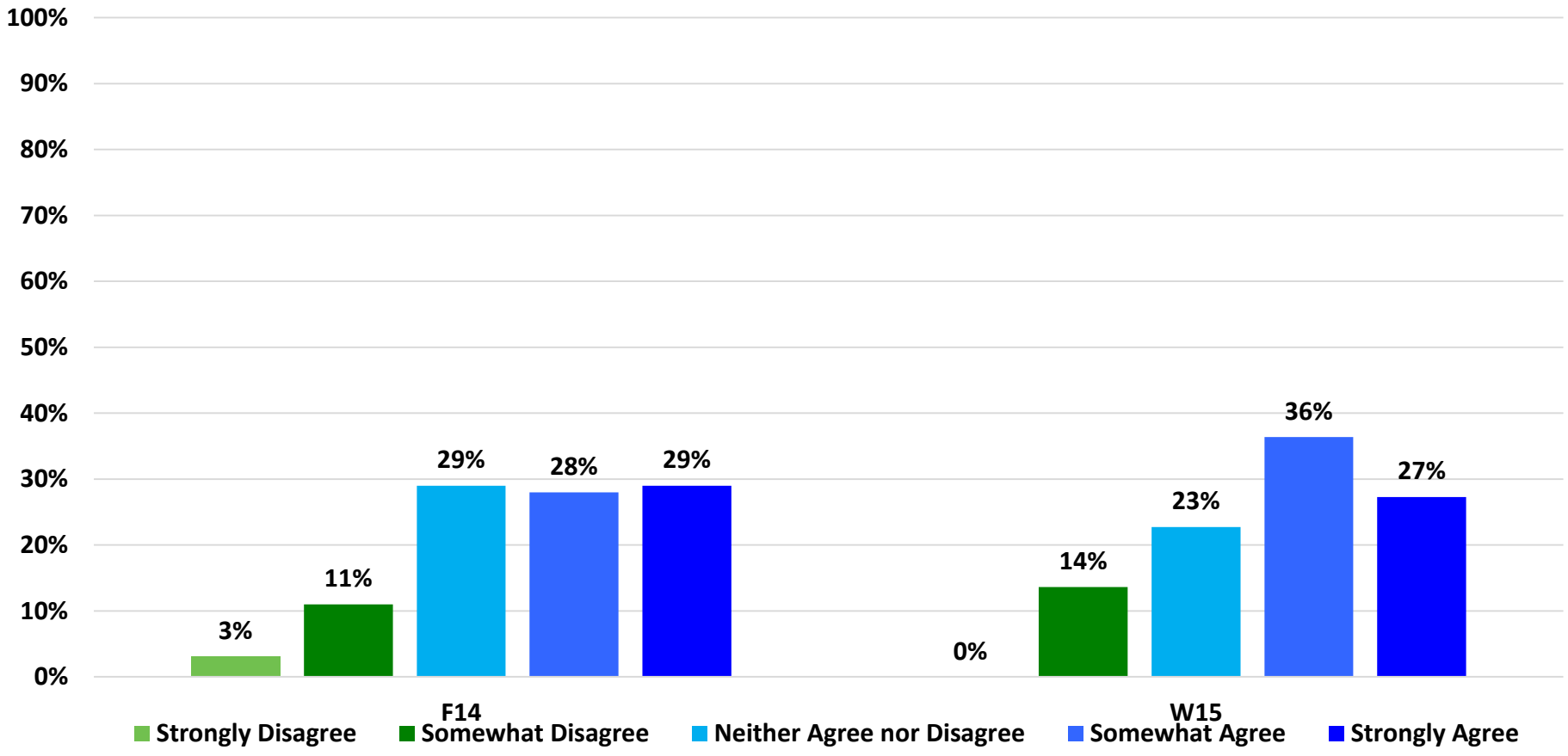
From Our Researcher Survey: Which of the following are reasons that you have not applied for PCORI funding? (N=182)



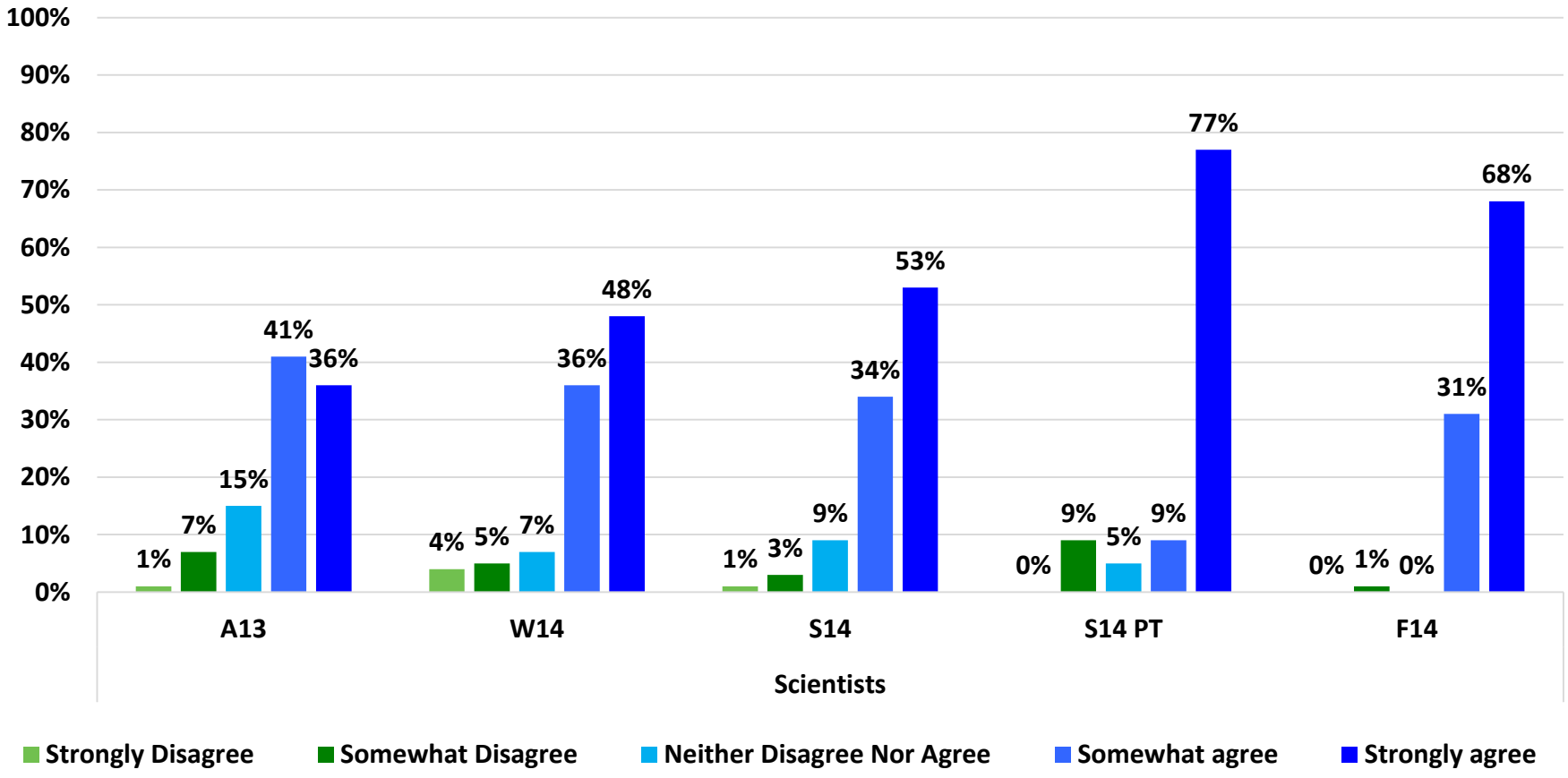
From Our Applicant Surveys:
I understood how to use the PCORI Methodology Standards to develop my research proposal



From Our Applicant Surveys:
Applying the PCORI Methodology Standards strengthened the scientific rigor of my proposed research

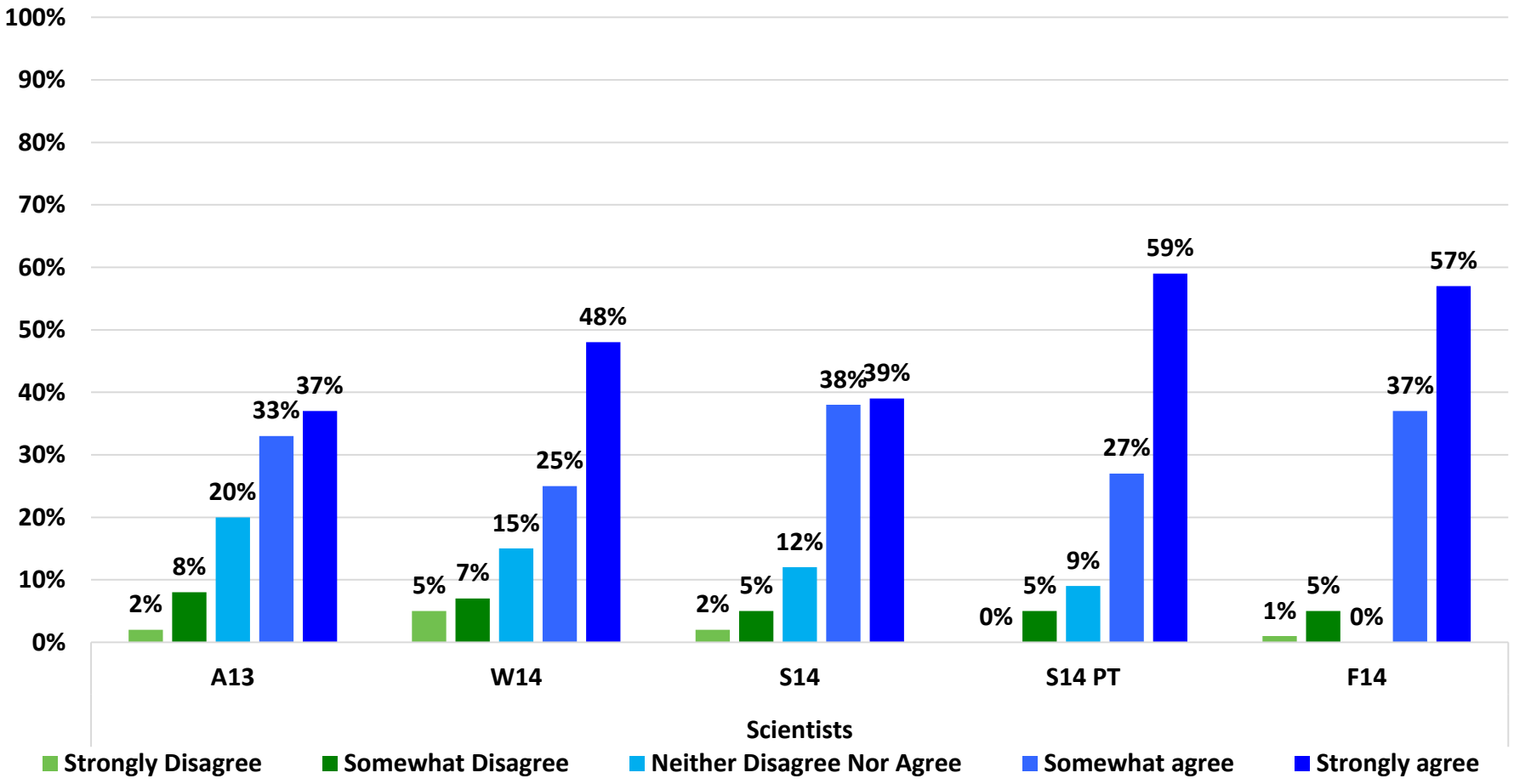


From Our Merit Reviewer Surveys:
I understood how to use the PCORI Methodology Standards to evaluate my assigned application



*Asked only of Scientist reviewers

From Our Merit Reviewer Survey:
The PCORI Methodology Standards were a useful resource for evaluating the technical merit of my assigned applications



**Asked only of Scientist reviewers*

FY2015 Financial Review (As of 2/28/2015)

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

Overview

- Summary
  - Revenue and Cash Balance
  - Research Contract Obligations
  - FY2015 Research Funding Commitments
- Budget vs. Actuals Review (as of 2/28/2015)
  - Variance Analysis
  - Cost Savings
  - Delayed Expenses
- Next Steps

Summary: PCORI Revenue and Cash Balance

Cash Balance at 9/30/2014	**$626.4 million**
Revenue from 10/1/2014-2/28/2015	**$251.7 million**
Federal Appropriation	*$120 million*
CMS Transfers	*$92.8 million*
PCOR fee	*$38.9 million*
Cash Disbursements	**$71.9 million**
Cash Balance at 2/28/2015 in PCOR Trust Fund and bank account	**$806.2 million**

Summary: Research Contract Obligations

Cumulative Obligation (Funding Commitment Made from inception to 2/28/2015)	$736 million*
Outstanding Obligations (Pending Payments)	$604 million

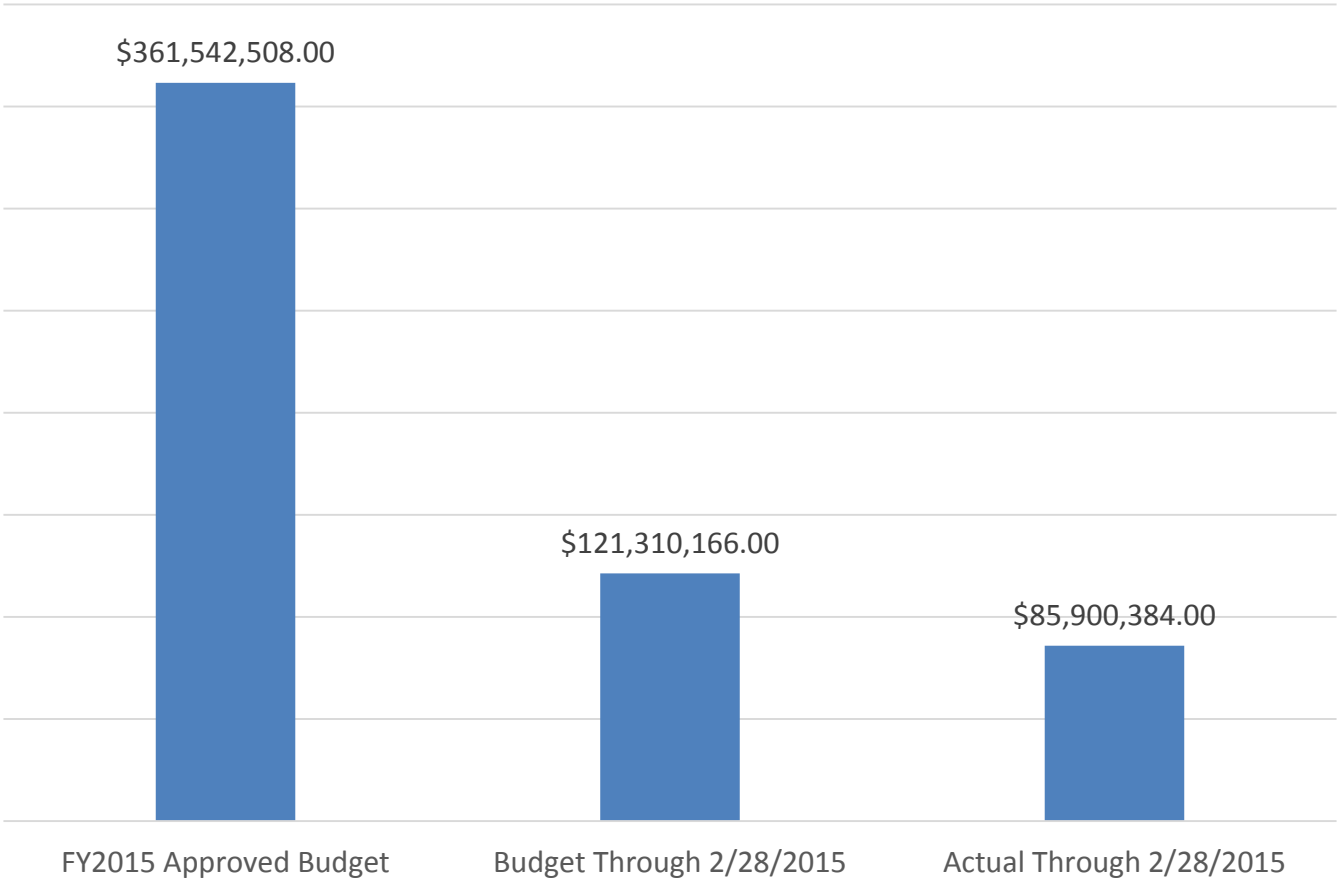
**$736M includes MOU with NIH on Falls and MOU with AHRQ on Uterine Fibroids*

Summary: FY2015 Research Funding Commitments

FY2015 Research Funding Awarded by BOG as of 2/28/2015	$64 million*
FY2015 Research Funding Commitment Plan (inclusive of the amount above)	$640 million

**$64M does not include Hypertension MOU of $25M or $1M Amendment to PCORnet Coordinating Center*

FY2015 Budget vs. Actuals
(As of 2/28/2015)



FY2015 Budget vs. Actual by Broad Categories (As of 2/28/2015)

Department	2015 Budget	Budget thru 2/28/15	Actual thru 2/28/15	Variance thru 2/28/15 ($)	Variance thru 2/28/15 (%)
Program Expense					
Research and Engagement Awards	271,906,381	85,452,687	63,500,877	21,951,810	25.7%
Program Support					
Methodology Committee	2,275,000	897,917	118,543	779,374	
Science/Program Development & Evaluation	34,857,973	14,044,378	6,708,519	7,335,859	
Engagement	11,529,494	4,687,445	3,066,407	1,621,038	
Contracts Management	11,307,062	4,874,490	2,437,503	2,436,987	
SUB TOTAL	***59,969,529***	***24,504,229***	***12,330,973***	***12,173,257***	49.7%
Administrative Support					
Board of Governors	1,250,000	490,417	538,682	(48,265)	
Management and General	28,416,598	10,862,833	9,529,852	1,332,981	
SUB TOTAL	***29,666,598***	***11,353,250***	***10,068,534***	***1,284,716***	11.3%
TOTAL	**361,542,508**	**121,310,166**	**85,900,384**	**35,409,782**	**29.2%**

FY2015 Budget vs. Actual Percentages by Broad Categories (As of 2/28/2015)

Department	2015 Budget	Percent of Total Budget	Actual thru 2/28/15	Percent of Actual Though 2/28/15
Program Expense				
Research and Engagement Awards	271,906,381	***75%***	63,500,877	***73%***
Program Support				
Methodology Committee	2,275,000	1%	118,543	0%
Science/Program Development & Evaluation	34,857,973	10%	6,708,519	8%
Engagement	11,529,494	3%	3,066,407	4%
Contracts Management	11,307,062	3%	2,437,503	3%
SUB TOTAL		***17%***		***14%***
Administrative Support				
Board of Governors	1,250,000	.3%	538,682	.6%
Management and General	28,416,598	8%	9,529,852	11%
SUB TOTAL		***8%***		***12%***
TOTAL	**361,542,508**	**100%**	**85,900,384**	**100%**

Key Factors in Variance - Cost Savings
(through 2/28/2015)

Key Factors in Variance	Amount ($)	Comment
Salaries and Benefits	$3.1 Million	Costing model assumes all new hires will start on day 1 of the quarter to assure we have funds for position when hired. Anticipate continuing to see underspend in this categories as the budget includes contingency for position adjustment, etc.
Merit Review	$900,000	Competitive LOI process has reduced the volume of application reviews; has led to smaller merit reviews; has led to in-house venue rather than external meeting space.
Other meetings	$500,000	Meetings in PCORI office space rather than external meeting venue and webinars by teleconference rather than webcast.

Key Factors in Variance – Delayed Activities (through 2/28/2015)

Key Factors in Variance	Amount ($)	Comment
Plans for two MOUs changed	$25 million	One topic was incorporated into the Pragmatic Studies and the second is slated to be issued in FY2016.
Methodology Committee	$780,000	Methodology curriculum contract has just been awarded. Discussion on a Priority Methods workshop have just begun.
Pipeline to Proposal related cost	$770,000	The complexities in negotiating and finalizing the contracts for intermediary regional offices delayed the Pipeline to Proposal program six months. These costs will carry over to FY2016 and beyond.
Pipeline to Proposal Awards	$718,000	
Training	$317,000	After a round of strategic review, Engagement identified two training programs and started negotiations with a vendor to provide CME/CE accreditation

Next Steps

- Incorporate latest cost assumptions into FY2016 budget planning
- Project additional research contract costs for modifications due to peer review requirements
- Project additional PCORI operating costs due to modifications from peer review

Updating PCORI's Process for Developing Scientific Publications

Bill Silberg
Director of Communications

Background

- Scientific Publications Subcommittee established in 2011/2012
- Charged with oversight of scientific articles* developed on PCORI's behalf
  - Approve manuscript ideas initiated by Board, MC or staff
  - Review external solicitations for papers written on behalf of PCORI
  - Identify appropriate Board, MC, or PCORI staff to author (if approved)
  - Ensure no overlap with papers already in development
- An internal management process would handle day-to-day aspects of developing papers

**Articles prepared for scientific journals and that undergo scientific peer review or editorial review*

Revisiting Our Process: Needs and Opportunities

- The volume of potential articles produced by PCORI staff and leadership is growing with the ramp-up of our activities
- Committee members' time, expertise and insight can be used more effectively than they are now
- Enhancing our scientific publishing planning can greatly advance our overall communications and dissemination efforts
- Improving our internal editorial process for developing articles in close collaboration with a reconstituted Committee will help to advance our strategic goals

Proposed Improvements

- Reconstituted Scientific Publications Committee, with new charter
- Updated scientific publication planning framework
- Revamped internal PCORI Editorial Group to manage development and submission of papers

New Committee Charter

- Members: up to four Board and up to three MC members, plus Executive Director/designee
- Advise and make recommendations on:
  - Enhancing PCORI's leadership in PCOR/CER through scientific publishing
  - Providing guidance for development and implementation of a scientific publishing strategy and internal process for developing scientific articles
  - Provide input on themes and topics to focus on in developing scientific articles
  - Provide content expertise as requested
  - Advise on conflict of interest and related issues that may arise in developing scientific articles

Next Steps

- Board consideration for approval of Scientific Publications Committee charter
- If approved, Board Chairperson may appoint Committee members
- Once appointed, Committee will convene to review publishing plan and internal editorial group proposed by staff
- Following review, staff will finalize plan, organize and implement editorial group
- Committee will convene regularly for updates and input

Board Vote

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

Public Comment

Break

We'll return at 1:15 p.m.

PCORnet Research Demonstration Projects

Rachael Fleurence, PhD
Program Director CER Methods and Infrastructure, PCORI

Joe Selby, MD, MPH
Executive Director

PCORnet Research Demonstration Projects

Initiative	Status
Aspirin Research Demonstration Project (Clinical Trial)	• Received Board approval for limited PFA development and funding on July 29, 2014 • **Seeking approval for the proposed project today**
Obesity Research Demonstration Projects (Observational Studies)	• Received Board approval for limited PFA development and funding on January 27, 2015
Health Systems Research Demonstration Projects	• Received Board approval for limited PFA development and funding on March 24, 2015
PPRN Research Demonstration Projects	• **Seeking Board approval for limited PFA development and funding today**
Natural Experiments in Translation – for Diabetes (NEXT-D)	• **Seeking Board approval for limited PFA development and funding today**

PCORnet Aspirin Demonstration Project Award Approval

Rachael Fleurence, PhD
Program Director CER Methods and Infrastructure, PCORI

Project Summary

- PCORI's first PCORnet research demonstration project
- Topic: Optimal Maintenance Aspirin Dose for Patients with Coronary Artery Disease
- Addresses an unanswered clinical question, one that matters to patients and clinicians
  - 53.6% of US patients with Coronary Artery Disease (15.4 million patients) are on high-dose aspirin (325mg), which may be associated with higher rates of gastrointestinal bleeding compared to low dose (81mg)
  - Variation in dose is driven largely by practice patterns rather than high quality clinical evidence
- A large multi-center trial would provide the necessary evidence to establish the relative effectiveness and safety of high dose vs low dose aspirin, influence clinical practice, and improve health outcomes for patients
- This trial will demonstrate PCORnet's capacity to support rapid and efficient randomized comparative effectiveness trials embedded in the delivery of care and leveraging electronic health data

Project Development

- Topic was generated from within the PCORnet entities, following a request for topics from PCORI in March 2014 (41 topics received)
- Working group consisting of Drs. Platt and Califf (PCORnet Coordinating Center) and Drs. Fleurence and Hickam (PCORI) identified six of these 41 topics that met PCORI criteria for this trial
- PCORI Advisory Panel on Assessment of Prevention, Diagnosis, and Treatment Options prioritized six topics
- PCORnet principal investigators discussed and ranked each of the six topics
- Both groups chose 'Maintenance dose for Aspirin' as the highest priority topic
- PFA development was approved by Science Oversight Committee June 24, 2014
- Topic was approved by the Board for PFA development on July 29, 2014

Application Review

- Application underwent rigorous technical and merit review that followed the principles of PCORI's review process for all research funding applications while taking into account the particular characteristics of PCORnet and this funding opportunity
- Application was reviewed by a panel of external merit reviewers including patients, researchers, and other stakeholders
- Following PCORI process, additional information was requested from applicants following merit review asking for study improvements, clarifications
- Clinical Trial Advisory Panel subcommittee comprised of merit review panelists, Clinical Trial Advisory Panel members, and other experts was formed to advise PCORI and participate in protocol refinement before contract execution as well as monitoring the trial on an as needed basis
- Received Selection Committee approval on April 29, 2015

Recommended Project

Project Title	Total Budget
Aspirin Dosing: A Patient-Centric Trial Assessing Benefits and Long-term Effectiveness (ADAPTABLE)	$14,016,506

Board Vote

Call for a Motion to:	• **Approve** the recommended PCORnet Aspirin Research Demonstration Project award
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

PCORnet PPRN Research Demonstration Project PFA Development

Rachael Fleurence, PhD

Program Director CER Methods and Infrastructure, PCORI

Project Background

- Approved PCORnet research demonstration projects focus on testing the capabilities of the CDRNs:
  - Aspirin Clinical Trial
  - Observational Studies on Obesity
  - Health System
- PPRNs need the opportunity to demonstrate their ability to conduct research **on behalf of their patient communities**
- PPRNs need to test their networks independently and across PPRNs
- Two initiatives (total costs not to exceed $22 million for up to nine projects in FY16):
  - Eight smaller projects at up to $2.5 million (total costs) over three years
  - One cross-PPRN project at up to $4 million (total costs) over three years

Project Scope

- PCORI will invite LOIs from PPRNs for studies that address patient-generated research questions
- Questions must be generated and approved by patient members
- Potential study designs must be optimal for the study question and might include health-related quality of life surveys, observational CER, interventional CER, patient preference studies, burden of disease studies, randomized controlled trials, methods studies, etc.
- There will be a collaborative aim for PPRNs, in addition to the scientific aim, to test the network's capacity to work together, similar to the CDRN-focused research demonstration projects:
    - Applicants will be asked to propose ways to leverage other PPRN knowledge, resources, and expertise and create re-usable processes for PCORnet

Proposed Timeline

- February 20, 2015: Research Transformation Committee endorsed limited PFA development
- March 17, 2015: Science Oversight Committee endorsed limited PFA development
- May 2015: Board considers approving limited PFA development
- May 2015: Limited PFA released*
- January 2016: Board considers approving slate of awards

*The PFA for the cross-PPRN project would be released later in Fall 2015 to allow enhanced collaborations between Phase I and Phase II PPRNs. This award would be considered by the Board in Spring 2016

Board Vote

Call for a Motion to:	• **Approve** the development of a limited PFA to fund up to nine PPRN research demonstration projects within PCORnet, not to exceed $22 million in total costs.
Call for the Motion to Be Seconded:	• **Second** the Motion • If further discussion, may propose an **Amendment** to the Motion or an **Alternative** Motion
Roll Call Vote:	• Vote to **Approve** the **Final** Motion • Ask for votes in favor, opposed, and abstentions

Natural Experiments in Translation – for Diabetes (NEXT-D)

Proposal to Support a Collaborative Project with the CDC and NIH using PCORnet

Joe Selby, MD, MPH

Executive Director

Project Background

- NEXT-D is a CDC and NIH-funded multi-center study based in five health systems now completing its first funding cycle and being re-competed
- Aim is to study the natural variation within and between systems in policies and clinical interventions aimed at preventing or managing diabetes mellitus – i.e., CER observational studies
- CDC has invited PCORI to join the CDC and NIH in co-funding the second cycle in order to add some EHR-based CDRNs participating in PCORnet to the five centers that will be funded by the CDC and NIH
    - PCORI funding would serve to expand the network beyond five sites by adding CDRNs, not substituting for CDC and NIH funds
- CDC/NIH will fund five sites at $450k/year and a coordinating center at $250K/year

Rationale for Participating

- Aligns well with PCORI's Principles of Collaboration
- Allows for collaboration among PCORnet sites: CDRNs could pool data or study variation between systems because of the Common Data Model
- Other features that could strengthen PCORnet sites:
  - Provides support for an active diabetes network that has sprung up among CDRNs in PCORnet (6 CDRNs applied to the CDC announcement)
  - Strengthens engagement of CDRNs with their delivery sites/plans, clinicians, and patients around diabetes care
  - Opportunity to demonstrate and expand PCORnet standards, Common Data Model to other systems and researchers
- Provides PCORI and entities participating in PCORnet with experience in analysis of natural experiments from delivery systems

Project Scope

- PCORI would fund up to three CDRNs beginning in FY16
  - $1.5 million/year (total costs)
  - Five year duration
  - $7.5 million total funding
- PCORnet CDRNs will apply to CDC
  - Five applications will be funded by the CDC and NIH
- PCORI could fund additional CDRNs based on PCORI merit review
  - To ensure comparability, PCORI will utilize CDC's technical review scores
  - PCORI will review CDRN applications for engagement and patient-centeredness
  - Applicants must comply with PCORI goals and requirements, including the methodology standards and methodology consultation
- Collaboration with CDC and NIH will take into account PCORI's collaboration principles and comply with PCORI's authorizing legislation

Proposed Timeline

- March 17, 2015: Science Oversight Committee endorsement for limited PFA development
- March 25, 2015: Research Transformation Committee endorsement for limited PFA development
- May 4, 2015: Board considers approving limited PFA development
- June 2015: PCORI limited PFA released
- September 2015: Board considers approving the slate of awards

Board Vote

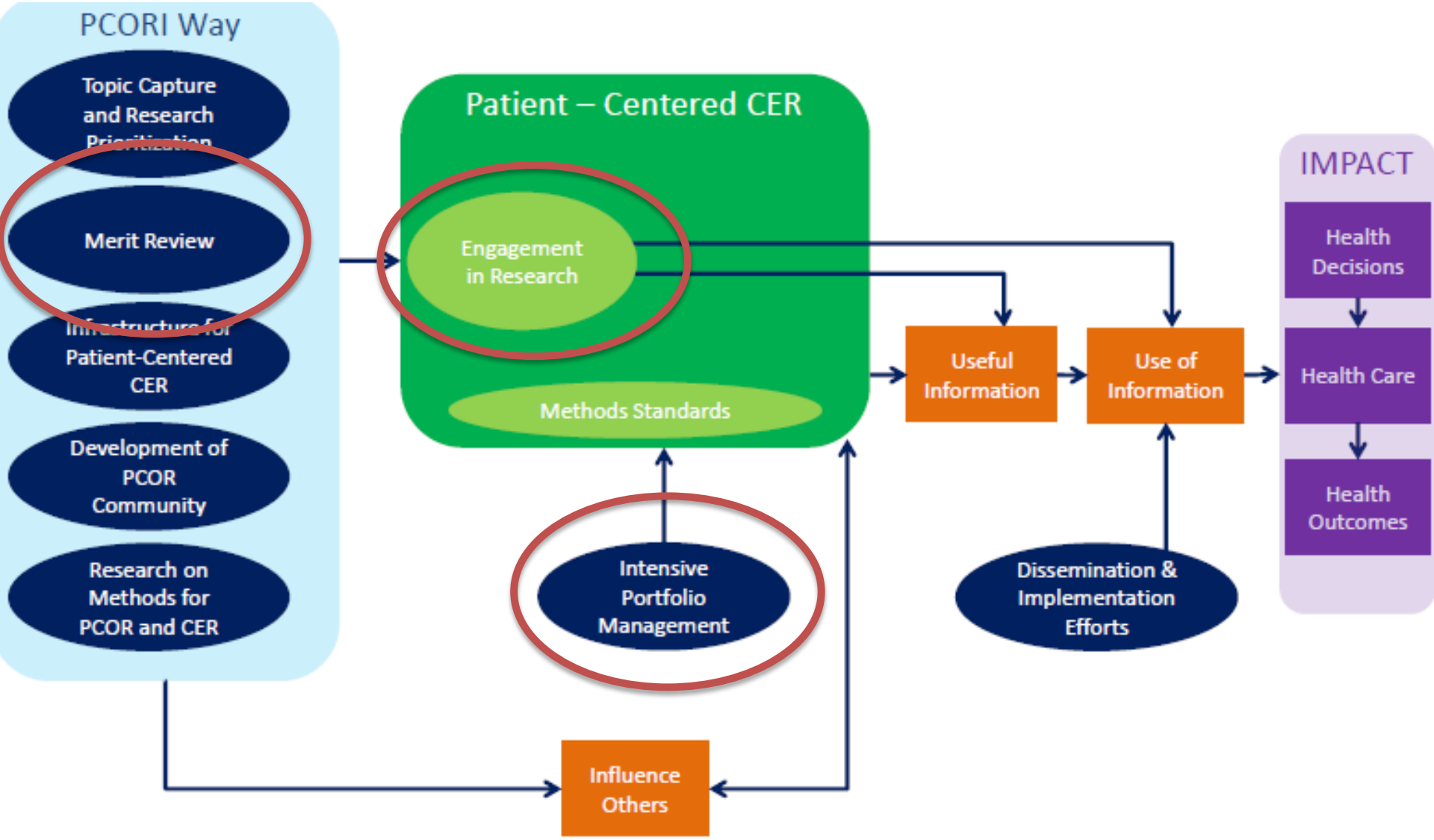
Call for a Motion to:	• **Approve** the development of a limited PFA to fund up to three CDRNs for participation in the CDC/NIH NEXT-D Network, not to exceed $7.5 million (total cost) over five years
Call for the Motion to Be Seconded:	• **Second** the Motion • If further discussion, may propose an **Amendment** to the Motion or an **Alternative** Motion
Roll Call Vote:	• Vote to **Approve** the **Final** Motion • Ask for votes in favor, opposed, and abstentions

Evaluation Update

Laura Forsythe, PhD, MPH
Associate Director, Evaluation & Analysis

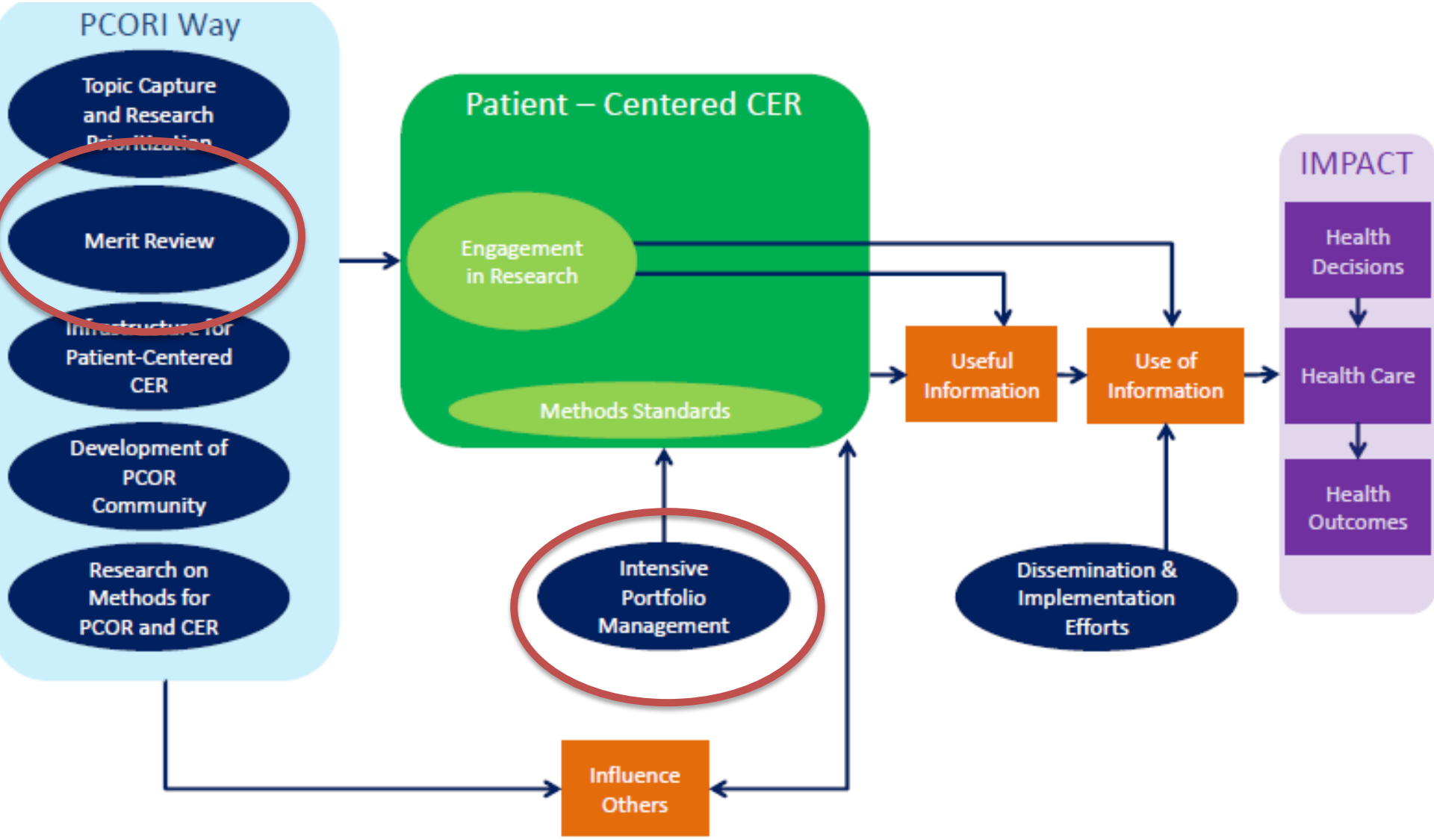
Lori Frank, PhD
Program Director, Evaluation & Analysis

PCORI Evaluation Framework



Analysis of Rare Diseases Applications

PCORI Evaluation Framework



Evaluation Objectives

- Understand whether success of PCORI applications on rare diseases differs from that of other applications, and if so, why
- Identify steps to support rare disease funding from PCORI

Evaluation Questions

- How many applications on rare diseases are reviewed, discussed and funded compared to other conditions?
- Compared to other applications, how likely are applications on rare diseases
  - to be discussed (i.e., part of the review slate at the in-person panels)? Why?
  - to be funded? Why?

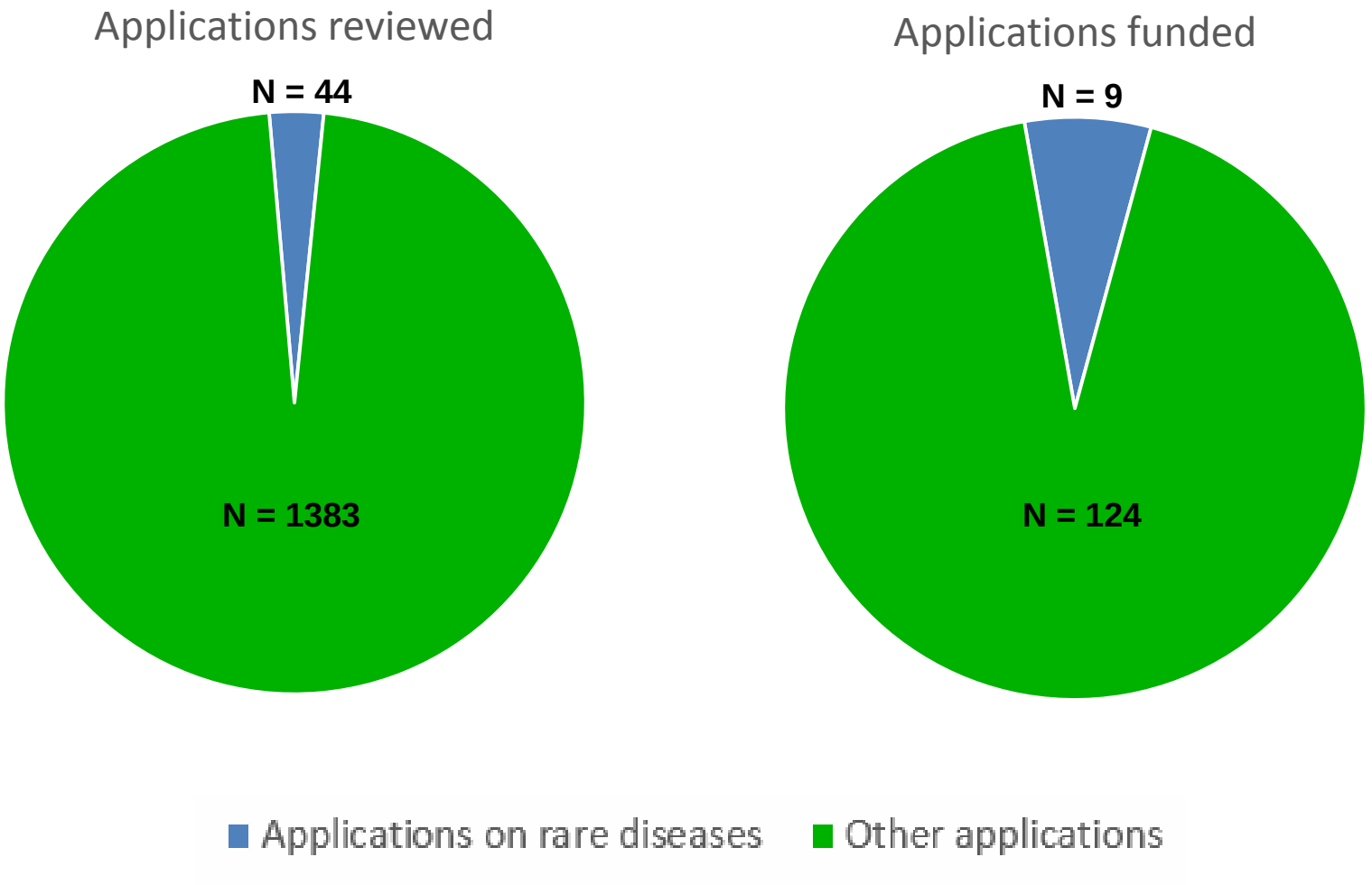
Funded Projects on Rare Disease

- Through April 2015, PCORI has 49 awards on Rare Diseases
  - 18 through Broad Funding Announcements (6%)
  - 3 Pilot Projects (6%)
  - 20 Networks (100% of Clinical Data Research Networks; 50% of Patient Powered Research Networks)
  - 5 Pipeline to Proposal awards (6%)
  - 3 Engagement awards (8%)

Methods

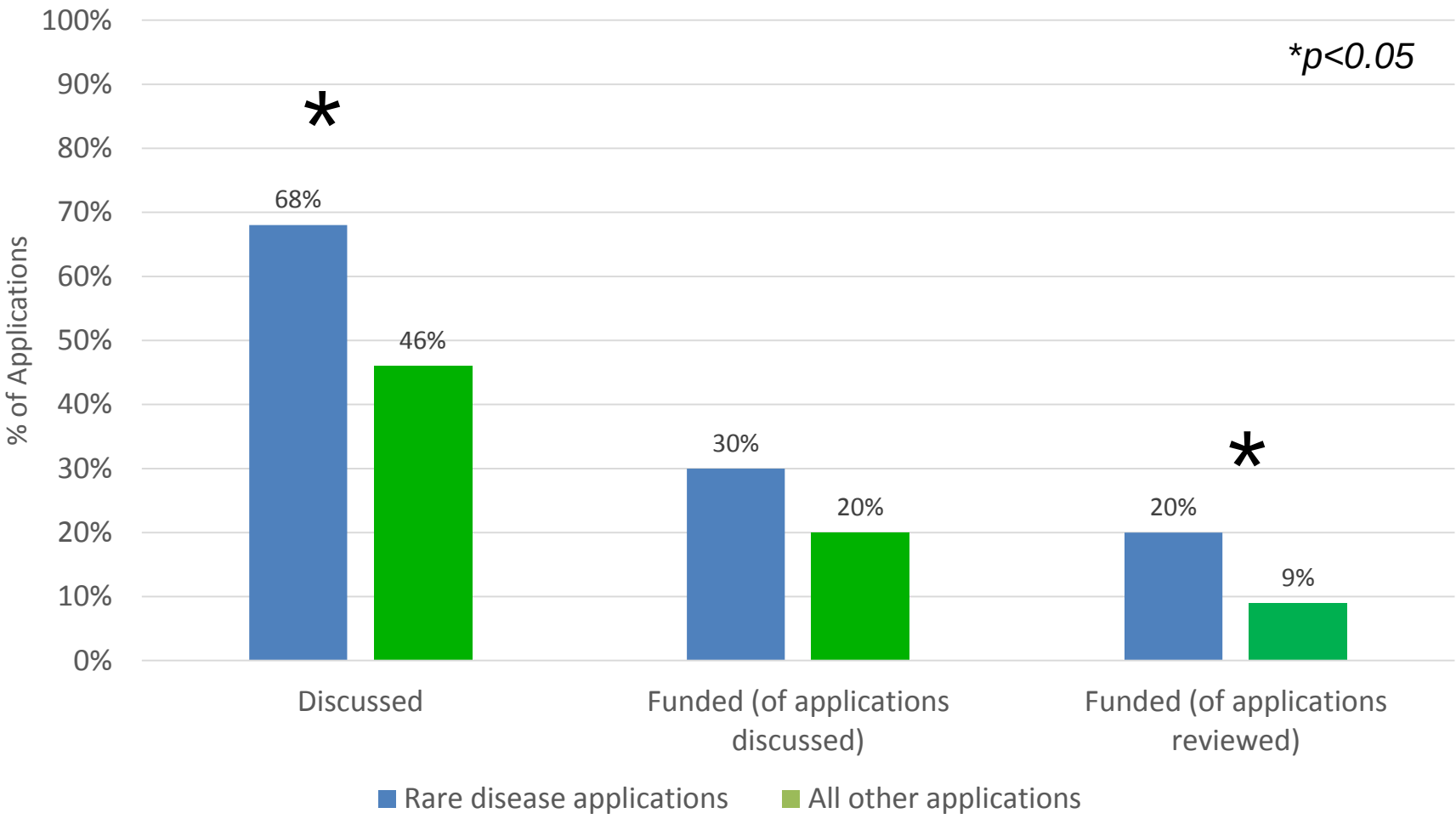
- Identified research proposals focused on rare disease
  - Submitted to broad PFAs (except Methods)
  - Cycles III (March 2013) through Spring 2014 (May 2014)
- Among those focused on rare diseases vs. all others
  - Examined the number reviewed, discussed, and funded
  - Compared the likelihood of discussion and funding
  - Compared criteria and overall scores, stratified by reviewer type

Applications Reviewed and Funded



Note: Broad PFAs (excluding Methods) Cycle III through Spring 2014

Likelihood of Discussion and Funding



Preliminary and Final Review Scores

- Applications on rare diseases scored similarly or better than applications on other conditions for each criteria and the overall scores
  - Scientist reviewers scored applications on rare diseases significantly more favorably for criterion 5 (Engagement)
  - Patient reviewers scored applications on rare diseases significantly more favorably for criterion 2 (Potential to improve healthcare and outcomes) and criterion 4 (Patient-centeredness)

Evaluation Summary

- Applications on rare diseases are not disadvantaged in PCORI Merit Review
- However, PCORI received a limited number of applications on rare diseases

Action Steps

- Set-aside funding for Rare Disease research in the Spring 2015 PFA ($12 M)
- Applications on rare diseases will be reviewed in separate panel(s) to ensure relevant experts are included

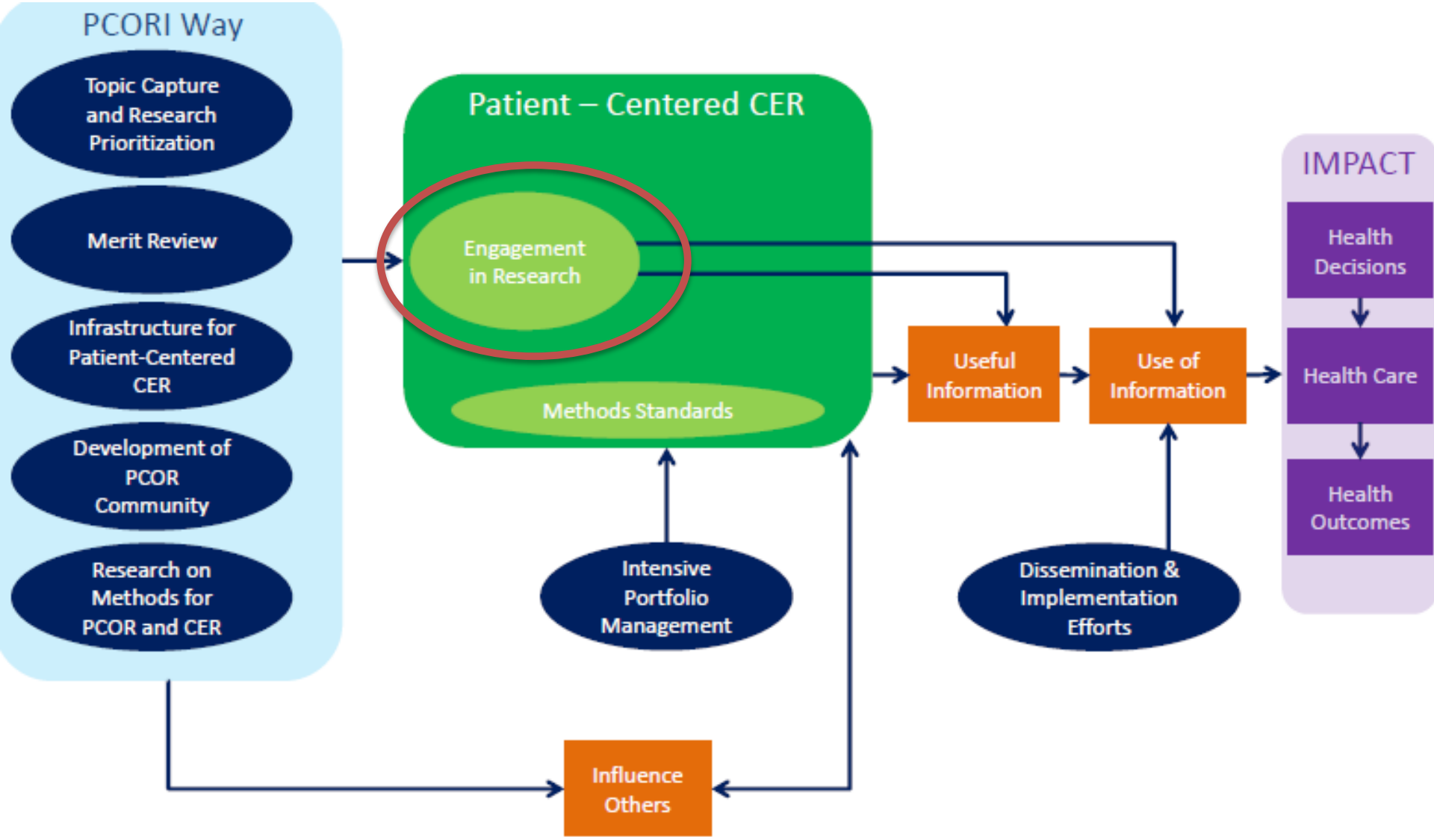
Receipt of Applications on Rare Diseases

- PCORI received 43 Letters of Intent (LOIs) on rare diseases for the Spring 2015 cycle
- 24 LOIs were invited to submit a full application
  - 56% of LOIs on rare diseases were accepted vs. 43% of other applications
  - LOIs on rare diseases account for 15% of accepted LOIs

Questions and Discussion

Understanding Engagement in Research

PCORI Evaluation Framework



Objectives for Measuring Engagement

- **Describe** engagement in PCORI funded projects
- **Evaluate** impact on PCORI strategic goals
- **Inform** PCORI funding requirements
- **Guide** current awardees, future applicants, and others interested in PCOR
- **Support** project progress based on learnings

Ways of Engaging - ENgagement ACtivity Tool (WE-ENACT)

- Pilot project awardees:
  Baseline and Project End
- Subsequent awardees:
  Baseline and Year 1

- Awardees nominate research partners to be invited to respond

WE-EnAcT Data Collection

	Researchers N (% response rate)	Patient/stakeholder partners N (% response rate)
Baseline	60 (82%)	97 (54%)
Year 1	99 (71%)	177 (56%)
End of project (Pilot projects)	27 (54%)	25 (56%)
TOTAL	186	299

Methods for Qualitative Analysis

(N=105 researchers, 93 patients and stakeholders)

- Partnership with American Institutes for Research
- Developed & applied codebook based on research questions and review of the open-text responses
- Identified major themes
- Mapped themes to conceptual model of PCOR[1]

[1] *Frank L, Forsythe L, Ellis L, Schrandt S, Sheridan S, Gerson J, Konopka K, Daugherty S. Conceptual and practical foundations of patient engagement in research at the patient-centered outcomes research institute. Qual Life Res. 2015 Jan 6.*

Qualitative Research Questions

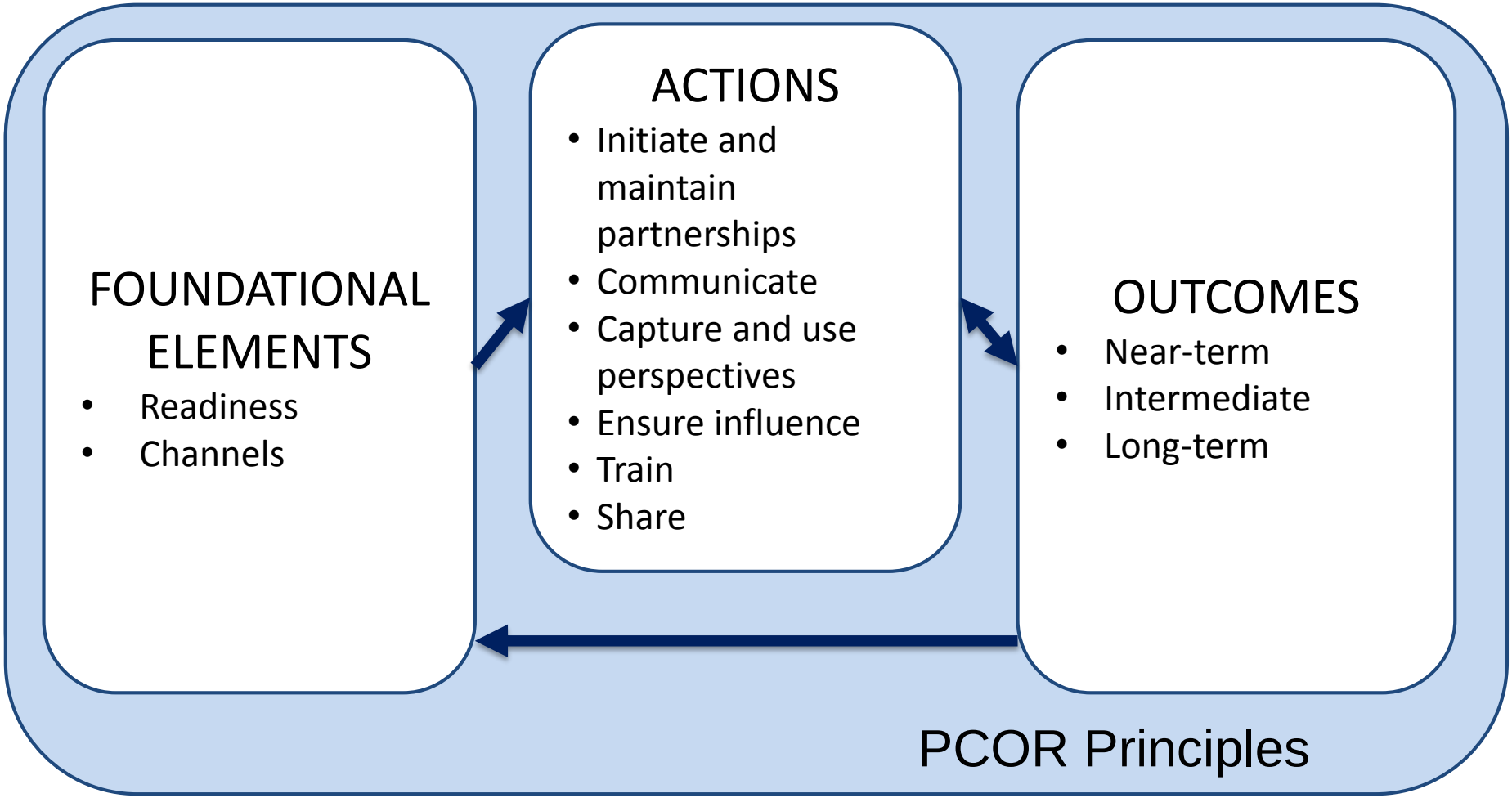
- Engagement strategies
- Barriers and facilitators
- Impact of engagement
- Differences by respondent type
- Questionnaire improvements

Data analyzed by content codes

Focus on codes with >25 responses

- engagement strategies,
- engagement impact,
- barriers,
- facilitators,
- how stakeholders got involved in the project, relationships,
- knowledge/training,
- logistical issues,
- PCOR principles

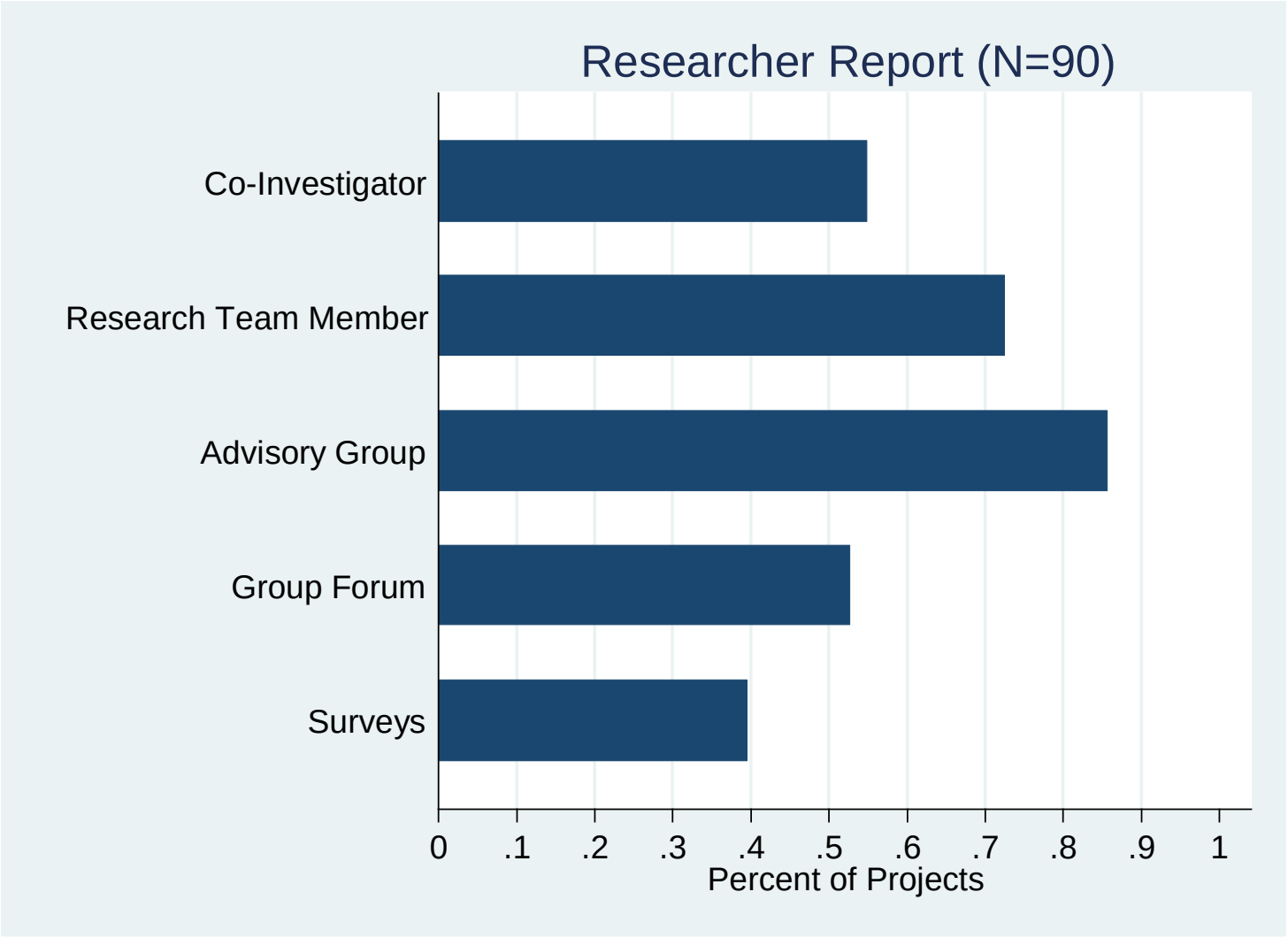
Conceptual Model of PCOR



Frank L, Forsythe L, Ellis L, Schrandt S, Sheridan S, Gerson J, Konopka K, Daugherty S. Conceptual and Practical Foundations of Patient Engagement in Research at the Patient-Centered Outcomes Research Institute Plans. *Quality of Life Research* 2015. 24(5):1033-1041 DOI 10.1007/s11136-014-0893-3

Results: Approaches to Engagement

Year 1



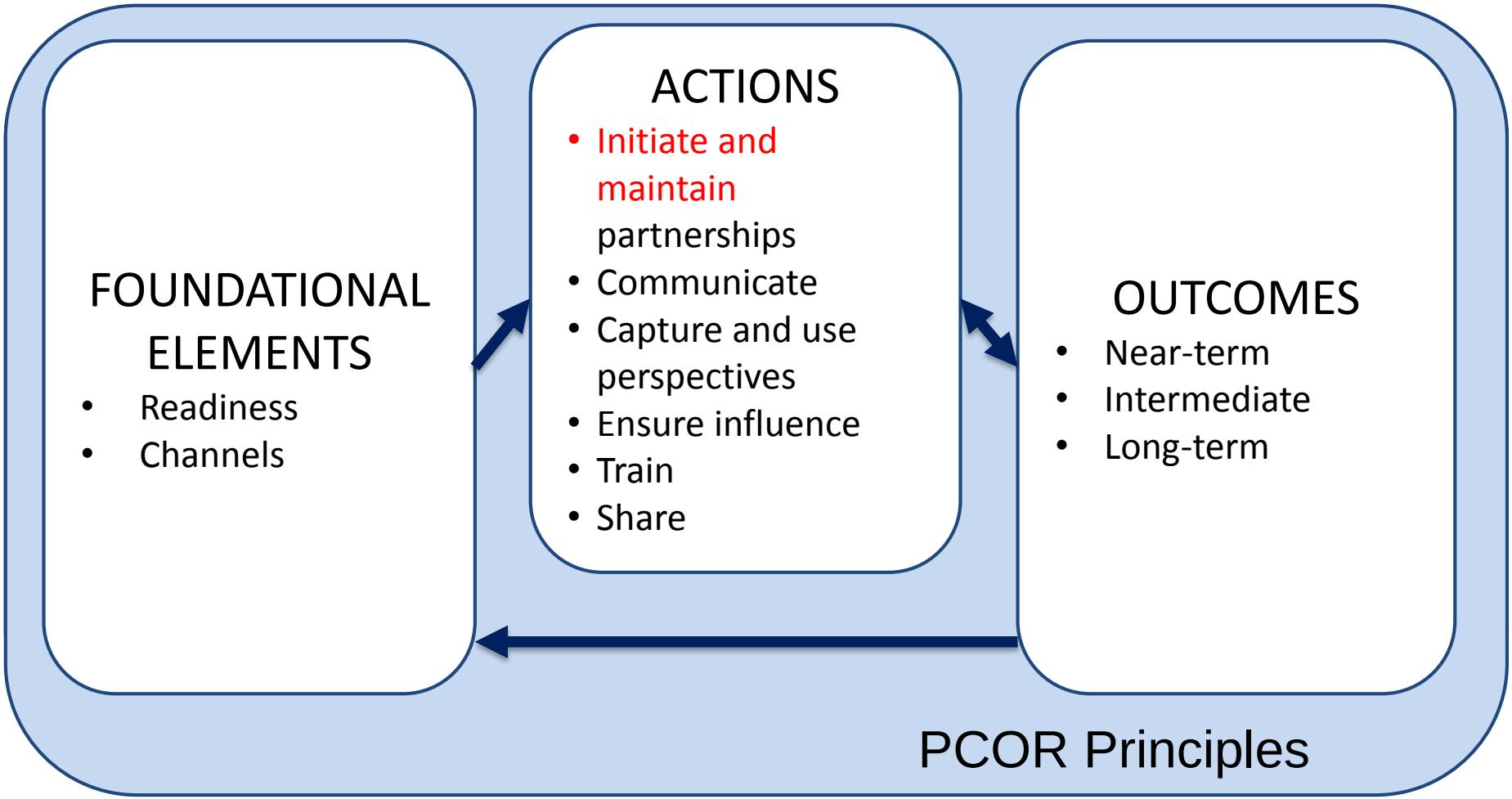
Results: Stages of Engagement

Year 1

Results: Type of Stakeholders Engaged

Year 1

Conceptual Model of PCOR



Results: Initiate and Maintain Partnerships
Early Engagement

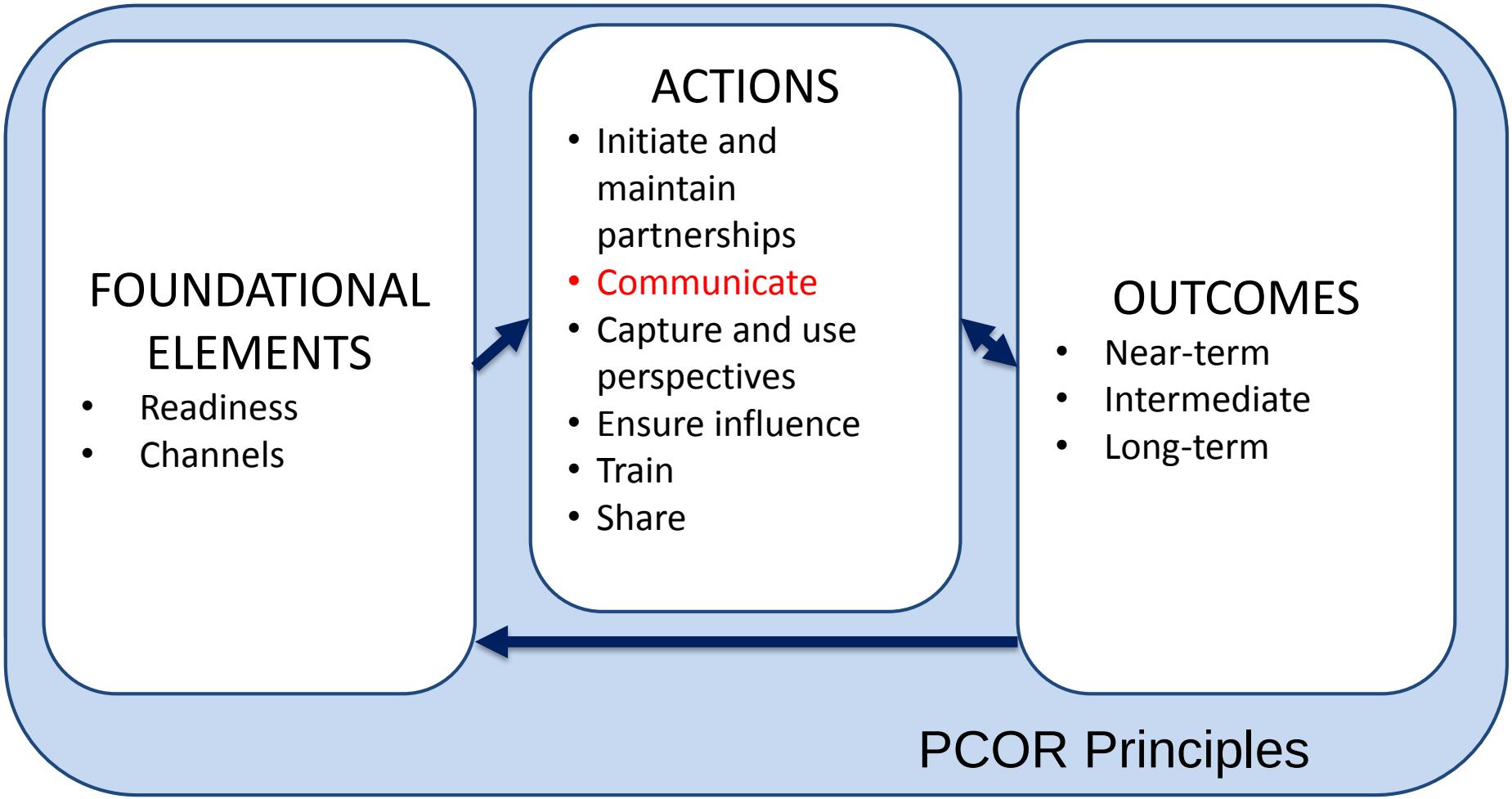
- Patients and stakeholders noted the usefulness of being involved early or experienced a desire to be involved earlier
- Researchers noted several challenges: keeping patients engaged throughout the project, setting expectations for project funding, and lacking funds for early involvement

"I wish they would have contacted us earlier in the grant process so we may have been able to work in more areas of the state vs. a small section."

"We did not have money to reimburse patients/stakeholders as we prepared the grant."

"It is always hard to go back to stakeholders…when a project has not been funded. This, in my opinion, is one of the greatest challenges to engaging with patients in the conceptualization and planning phases."

Conceptual Model of PCOR



Results: Communication
Creating an Open Environment for Sharing

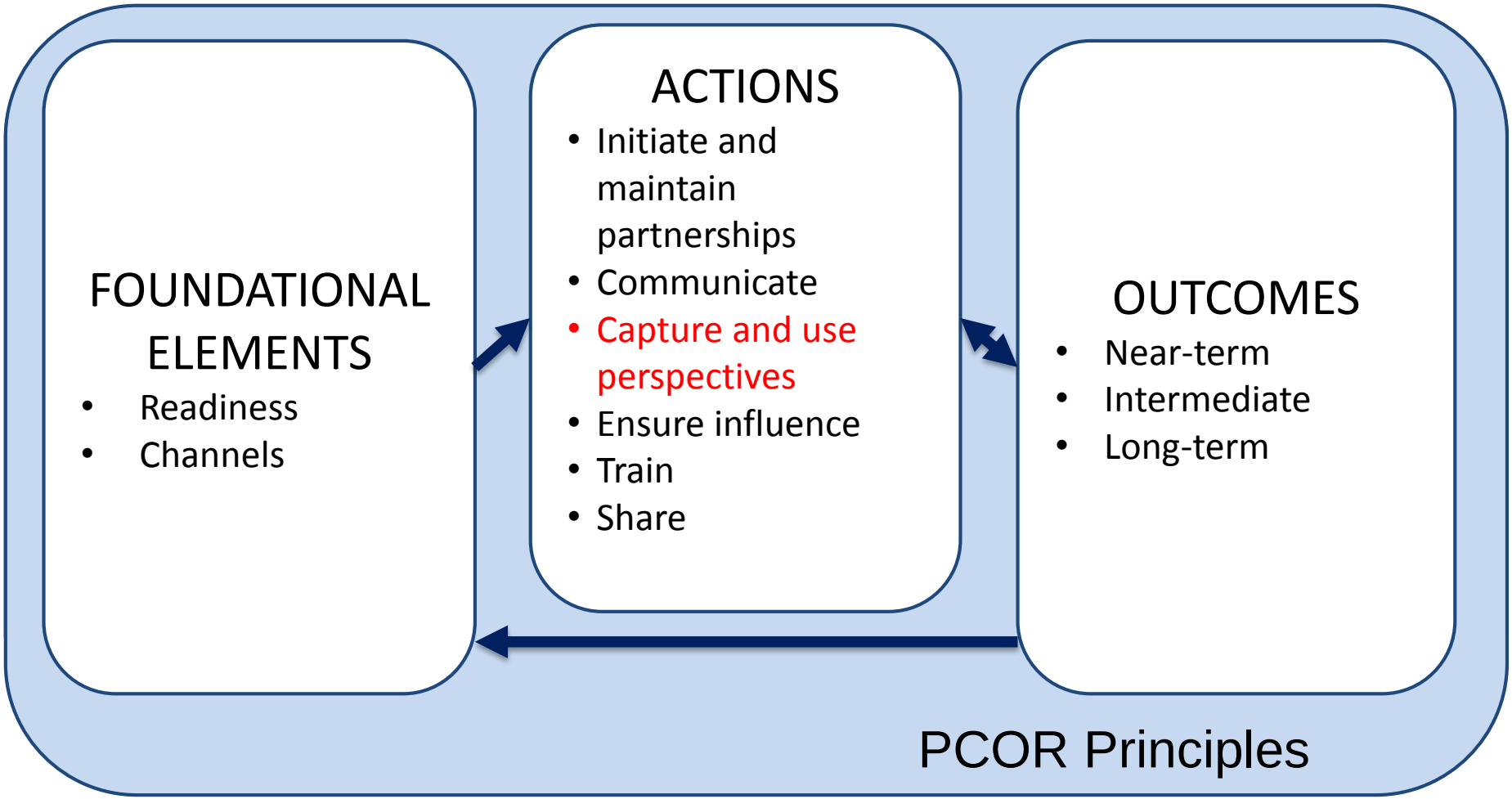
- Managing power differentials
- Managing diverse groups and culturally-sensitive interactions
- Using plain language

"We are still working on whether all the stakeholders should meet together, or if patients may not want that. It might be intimidating or inhibiting. How does one facilitate conversations across different stakeholder groups when there are strong feelings that can conflict? "

"Researchers need to understand patients and how to communicate with them, especially if they are not in the same age group or cultural background."

"It is sometimes difficult to "speak the same language" at group meetings. In other words, the language style tends to be dominated by researchers or clinicians."

Conceptual Model of PCOR



Capturing the Patient Perspective: Study Design

- Help choose research methods: measures, interventions, comparators, and outcomes
- Decide between data collection methods (e.g., in-depth interviews vs. focus groups)
- Review and revise study plans and materials

"Helped the investigators decide what cohort of patients to include."

"Gave clinical input into choice of screening measures."

"We presented aspects of study design to the group and solicited their input, i.e. - what should the "control group" be for the RCT - is it "standard practice" (which is no specific intervention in our topic) or should it be a currently existing but potentially ineffective intervention."

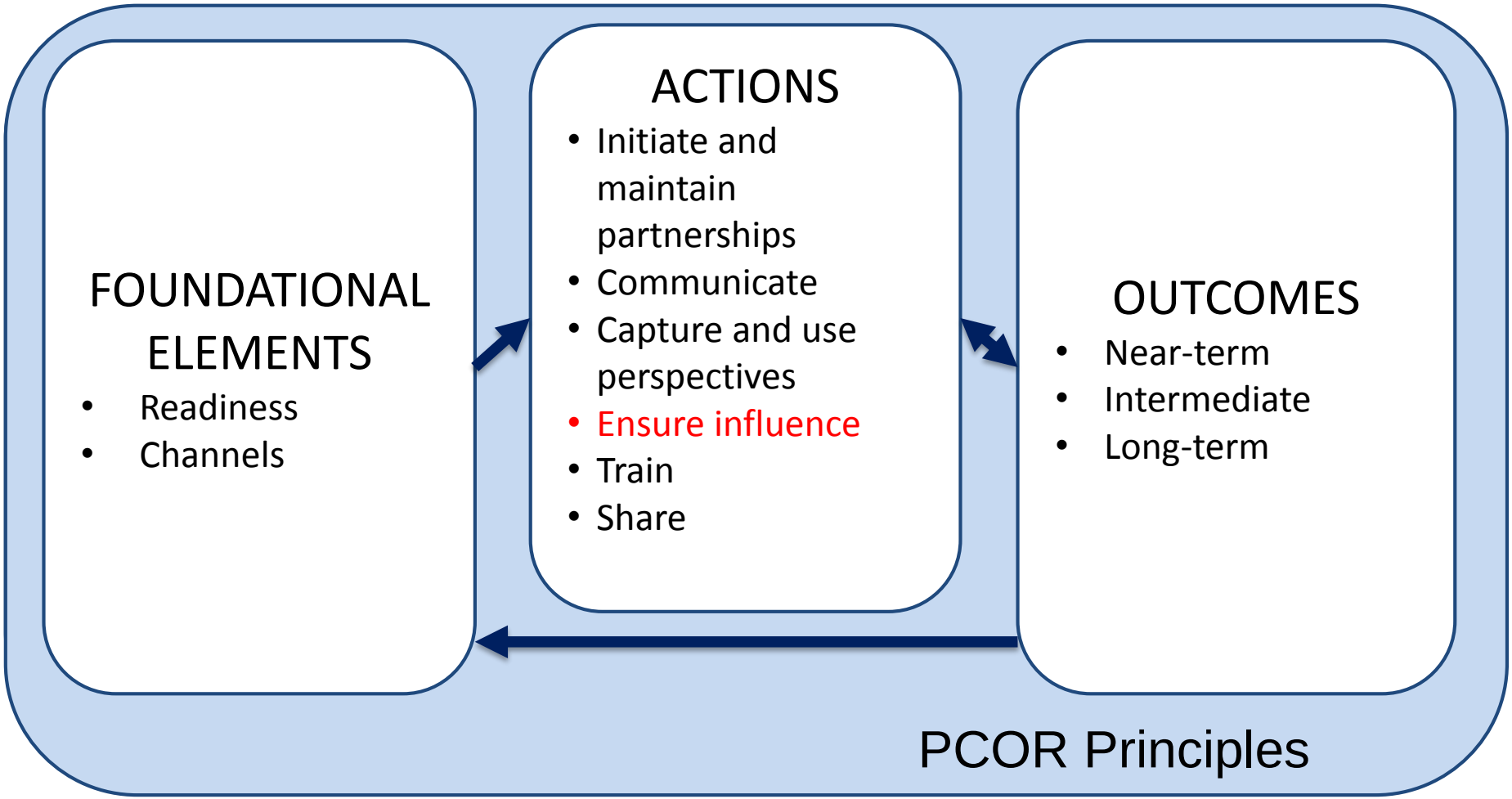
Capturing the Patient Perspective: Recruitment & Retention

- Strategize for recruitment and retention
- Help prepare for recruitment (e.g., developing materials)
- Serve as liaison between research team and groups to be recruited
- On-the-ground recruiting of study participants, practices, and partner organizations

"We helped the researchers to understand potential barriers to enrollment, particularly for minority candidates, and identified responses to these barriers."

"The stakeholder Co-I's relationship with individuals similar to those recruited for this study allowed her to provide insights to this population that is often difficult to recruit and maintain over the course of the study."

Conceptual Model of PCOR



Ensure Influence: Study Design

- Changes to study design to make it more responsive to patient needs, feasible in clinical setting
- Some researchers reported minimal impact on study design

"High impact - changed design, outcomes, flow of study."

"This led us to modify our original 2-group research design and include a 3rd group; community based group exercise."

"Contributed to the approach taken and to creating conditions that would allow maximum participation on the part of both patients and providers."

"The timeline of study assessments was modified in response to stakeholder feedback."

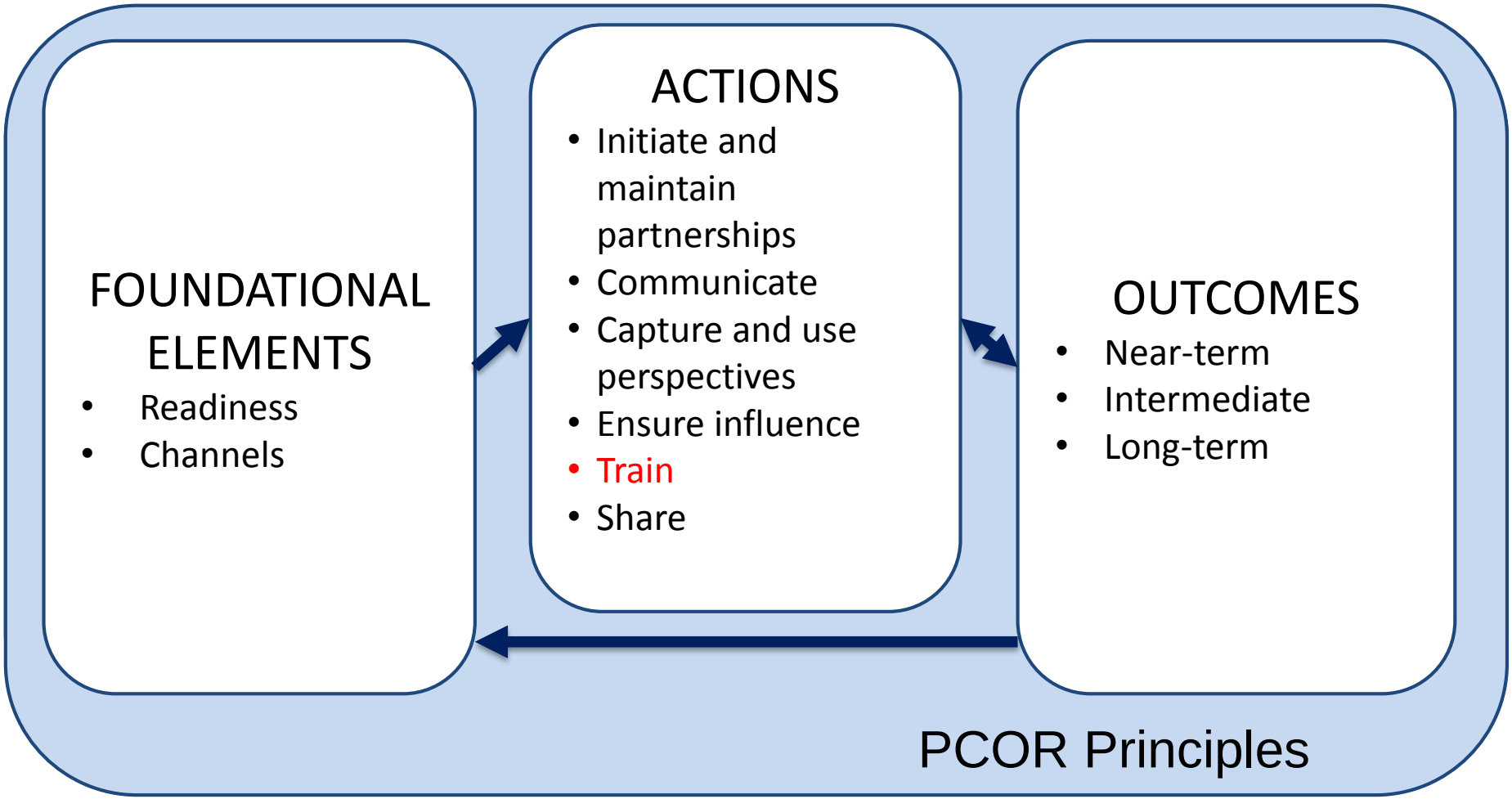
Ensure Influence: Recruitment & Retention

- Recruitment procedures more responsive to patient needs
- Changes to recruitment messages
- More potential participants aware of the study
- Improvements in recruiting and retaining difficult-to-reach populations

"Since discussing our challenges with recruiting and retaining study participants, we have had only one participant decline to participate."

"Outreach materials, recruitment procedures were modified significantly."

Conceptual Model of PCOR



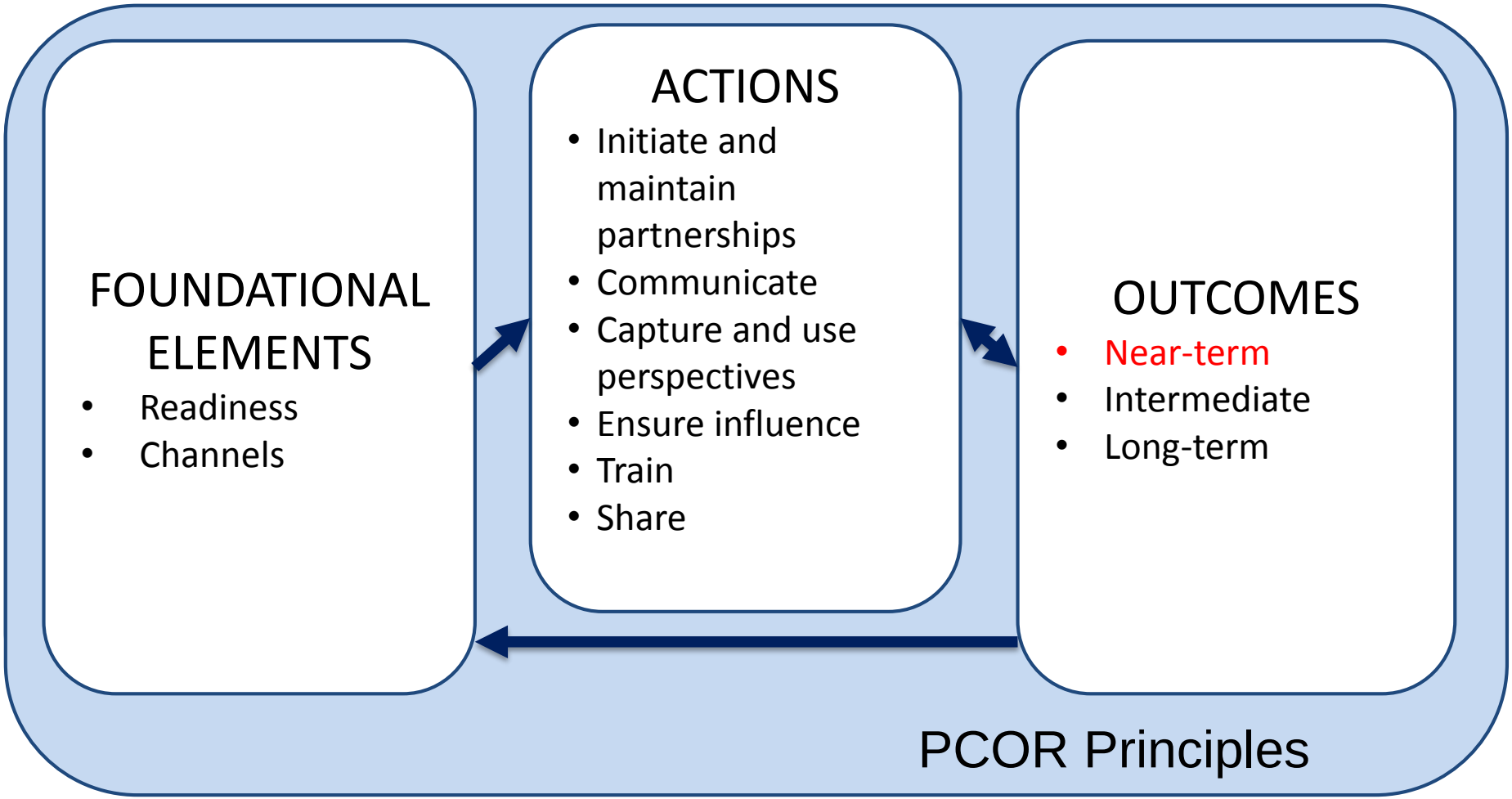
Results: Training for Partnership

- Importance of training
- Training needs
  - Topic background
  - Research methods
  - How to provide input effectively
  - How to communicate about research
  - Training for researchers on how to engage partners

"I have searched for training webinars and other tools to help…my staff to better understand our role."

"There is a steep learning curve to understanding research and how to conduct research."

Conceptual Model of PCOR



Early Impacts Beyond the Project - 1

- Increased knowledge and skills about research
- Increased knowledge about or engagement in health

"I have watched my staff improve in their professional skills as well through this project… We have learned skills and developed tools that will enhance our success in the future."

"The work on this project did inform me of the importance of patient/ family engagement in health care decision-making and has prompted me to adopt some of these approaches in my personal life."

Early Impacts Beyond the Project - 2

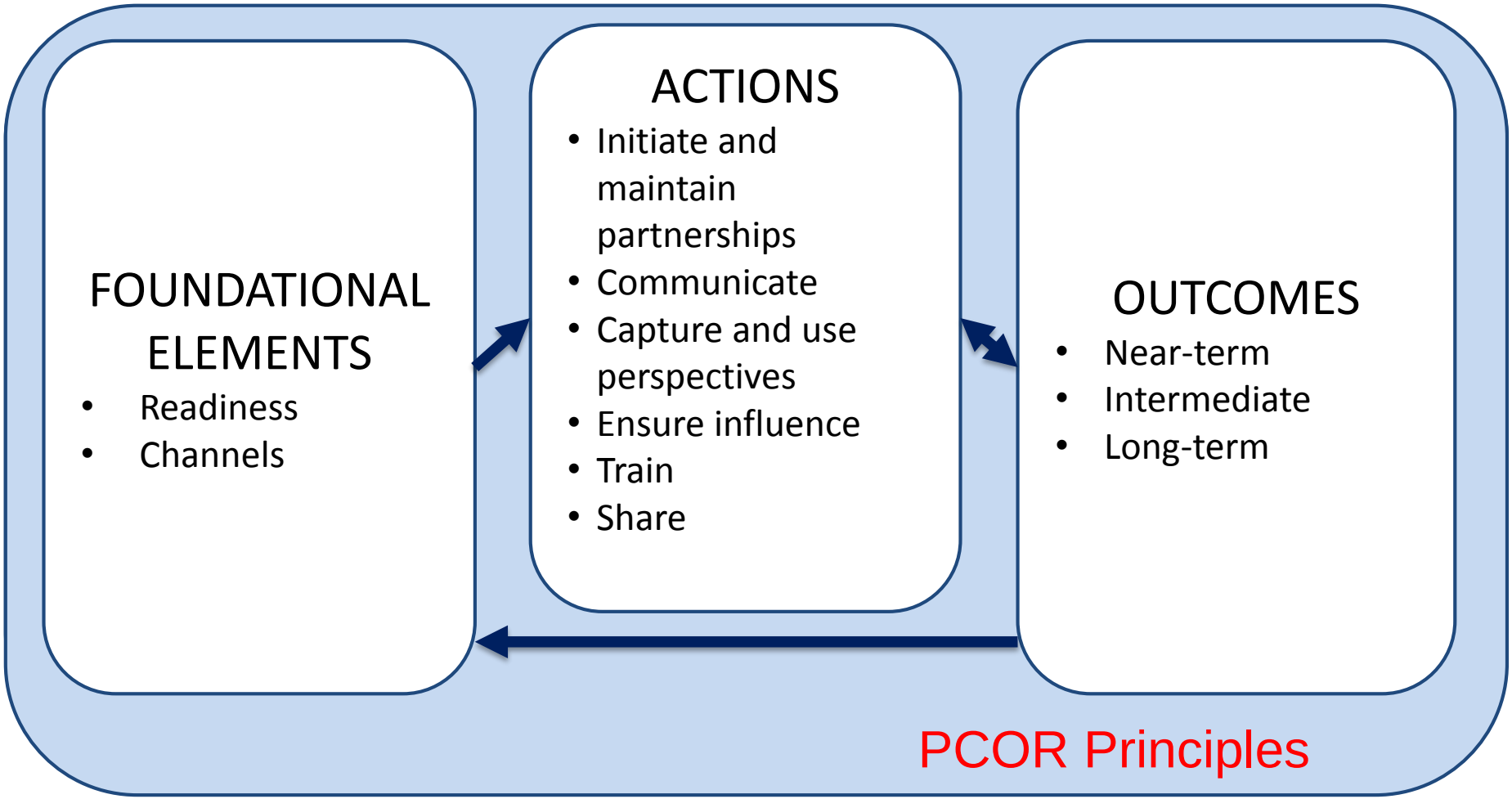
- Increased interest in patient/ stakeholder engagement
- Feeling like their participation had a larger impact

"I am more comfortable suggesting inclusion of patients on research projects."

"It allowed me to feel like a more rounded physician because I am doing research to help the general community. It expands my influence on the community in which I live."

"Expanded our interest and relationships with other researchers in our community… We also brought together several of the researchers in our community to discuss who we are and what we do as to try and coordinate projects geared towards senior adults."

Conceptual Model of PCOR



Conclusions

- Challenges during application phase include uncertainty about viability of partnership
- Impacts on study design ranged from none to major design changes
- Recruitment methods an important area of engagement impact
- Training needs identified by most respondents
- Early evidence for the impact of engagement across stages of the research

- Report on challenges at all stages and ways awardees addressed challenges are being incorporated in ongoing guidance

Turning information into action

- Application phase: expectations about application success, compensation
- Research relationships: in-person meetings, technology supports, protected time
- Opportunity for expanding stakeholder relationships
- Training: inclusiveness, managing groups, communicating expectations, incorporating input
- Facilitating sharing of learnings across awardees

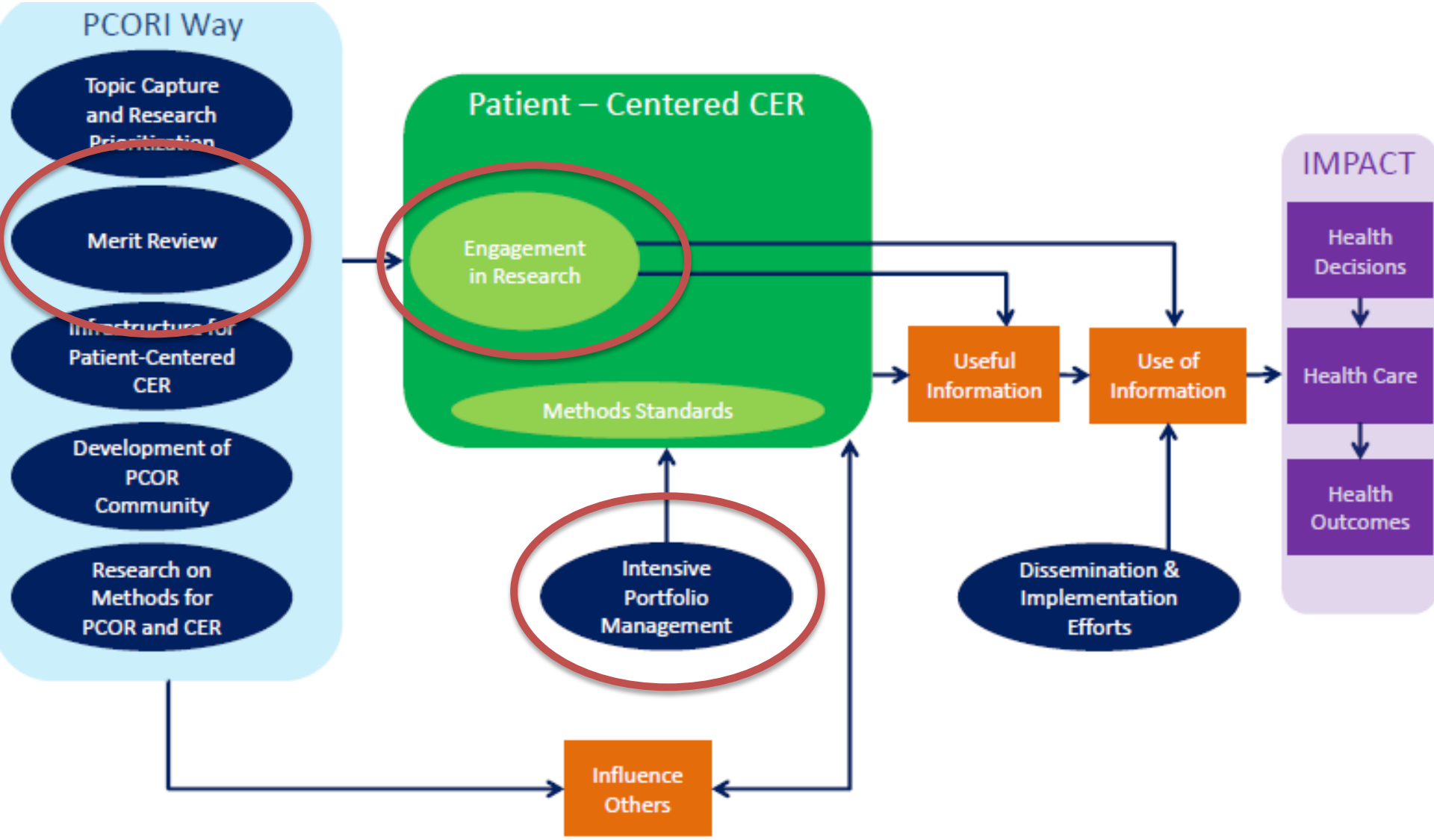
Sharing Findings

March 2015 webinar with awardees

Inventory of engagement activities

Planning the study
• Identifying research topics • Developing research questions • Proposal development • Developing the budget • Expanding the research team • Study design
Conducting the study
• Recruiting & retaining participants • Data collection • Data analysis • Results review, interpretation & translation
Dissemination
• Dissemination/ sharing research findings

PCORI Evaluation Framework



Questions and Discussion

Break

We'll return at 3:30 p.m.

Methodology Committee Update

Robin Newhouse, PhD, RN

Chair of the Methodology Committee

Session Topics and Objectives

What are we going to cover today?

Overview	• 18-month priority goals in categories of Methodology Committee activities
Generate Methodology Standards	• Create standards for designs using clusters • Create standards for research on complex interventions • Review/revise Methodology Standards
Identify Methods Gaps	• Identify and prioritize methods development opportunities for PCORnet • Develop "usual care" definitions and components • Conduct a "deep-dive" into health care decision science including research on patient preference • Develop activities and workshop around PROs in EHRs
Disseminate Methodology Standards	• Augment/refine Methodology Standards training and implementation
Other Updates	• GAO Nominations • Methods Consultation

Overview

- Since the last meeting, the MC has decided on their 18-month priority goals
- Using the revised Methodology Committee Charter as a guide, the MC developed categories of activities that contain eight priority goals. One category (Provide Methods Guidance) currently does not have identified goals

Generate Methodology Standards	Create standards for designs using clusters
	Create standards for research on complex interventions
	Review/revise Methodology Standards
Methods Gaps	Identify and prioritize methods development opportunities for PCORnet
	Develop usual care definitions and components
	Conduct a "deep-dive" into health care decision science including research on patient preference
	Develop activities and workshop around PROs in EHRs
Disseminate Methodology Standards	Augment/refine Methodology Standards training and implementation

Create Standards for Designs Using Clusters

- **Current Status**
  - Designs Using Clusters meeting occurred on April 7th with experts, MC members and PCORI staff in attendance
  - PCORI staff is actively revising a list of 8 draft standards developed during the April 7th meeting
- **Next Steps**
  - Staff workgroup will continue revising the draft standards
  - Draft standards will be presented to the MC on May 6 with proposed final approval at the Fall F2F
- **MC Members:** Naomi Aronson, Cynthia Girman, Robert Kaplan, Sally Morton, Robin Newhouse, and Sebastian Schneeweiss
- **Projected Timeframe:** 2014 – Fall 2015

Create Standards for Research on Complex Interventions

- **Current Status**
  - Created a work group consisting of staff and MC members
  - Developed search strategy for literature review
- **Next Step**
  - Staff work group will conduct literature review including definitions, scope, problems, current standards, and guidance statements related to research on complex interventions
- **MC Lead:** Brian Mittman
- **MC Members:** Naomi Aronson, David Flum, Robin Newhouse, and Mary Tinetti
- **Projected Timeframe:** February – Fall 2015

Review Existing Methodology Standards

- **Current Status:**
  - Four of eleven categories of standards are currently under review
    - Standards for Formulating Research Questions (RQ)
    - Standards Associated with Patient-centeredness (PC)
    - Standards for Heterogeneity of Treatment Effects (HTE)
    - Standards for Causal Inference Methods (CI)
- **Next Steps**
  - Review the remaining seven categories over the next five months
  - All reviewed/revised standards will be presented to the MC for approval at the Fall F2F meeting
- **MC Lead:** Each standards category has one or two MC leads
- **Projected Timeframe:** November 2014 – Fall 2015

Identify and Prioritize Methods Development Opportunities for PCORnet

- **Current Status**
  - Established MC work group with staff support
  - Identified methodological priorities
- **Next Steps**
  - Plan two workshops on missing data to occur in the Fall 2015
  - Identify additional staff to help with this initiative
- **MC Members:** Cynthia Girman, Steven Goodman, Sally Morton, and Sebastian Schneeweiss
- **Projected Timeframe:** Ongoing

Develop "Usual Care" Definitions and Components

- **Current Status**
  - Established MC work group with Harold Sox as staff lead
- **Next Steps**
  - Develop clear statement on "usual care"
  - Consider moving topic forward by writing an article for the *Annals of Internal Medicine*
- **MC Members:** Naomi Aronson, Ethan Basch, Mark Helfand, David Meltzer, Neil Powe, and Mary Tinetti
- **Projected Timeframe:** In development

Conduct a "Deep-dive" into Health Care Decision Science Including Research on Patient Preference

- **Current Status**
  - Contract in place
  - Experts identified and invited to June meeting
  - MC work group, staff, contractors, and experts have had several meetings over the last six weeks
- **Next Step**
  - Continue planning for workshop in early June and a larger meeting in Fall 2015
- **MC Leads:** David Flum, Mark Helfand, and David Meltzer
- **Projected Timeframe:** November 2014 – December 2015

Develop Activities & Workshop Around PROs in EHRs

- **Current Status**
  - MC work group identified
  - Goal is to plan a follow up workshop to the first PRO workshop that occurred November 2013
  - Identified link between PROs and PCORnet activities
- **Next Steps**
  - The group will help shape the workshop including overseeing the agenda, deciding on participants, and determining deliverables
- **MC Lead:** Ethan Basch
- **MC Members:** Naomi Aronson, David Flum, Cynthia Girman, Robert Kaplan, Neil Powe, and Mary Tinetti
- **Projected Timeframe:** In development

Augment/Refine Methodology Standards Training and Implementation

- **Current Status**
  - Baylor College of Medicine CME/CE Methodology Standards educational activities planned for end of May
    - MC members are involved in developing modules
  - Methodology Standards Curriculum development in progress
    - Contract awarded April 1 and is under way
- **Next Step**
  - MC to help determine organizations for promotion of CME/CE activities
  - Delivery for Methodology Standards Curriculum expected September 2015
- **MC Lead and Members:** To be determined
- **Projected Timeframe:** Ongoing

Other Updates

- GAO nominations for the final position on the Methodology Committee (health informatics expert) closed on November 17, 2014—Pending Appointment
- Methods Consultation Panels for Pragmatic Clinical Studies (PCS)
  - Held Methods Consultation Panels for first 2 cycles of PCS and will continue for future cycles
  - Preliminary evaluation showed that it added value and will continue further evaluation
  - MC Lead: Steve Goodman

Methodology Committee Members

- Robin Newhouse, Chair
- Steven Goodman, Vice Chair
- Naomi Aronson
- Ethan Basch
- David Flum
- Cynthia Girman
- Mark Helfand
- Robert Kaplan
- Michael Lauer
- David Meltzer
- Brian Mittman
- Sally Morton
- Neil Powe
- Sebastian Schneeweiss
- Mary Tinetti
- Clyde Yancy

Communication and Dissemination Research Program

Jean Slutsky, PA, MSPH

Chief Engagement and Dissemination Officer and

Program Director, Communication and Dissemination Research

Presentation Outline

- CDR team introductions
- Portfolio overview
- 2015 CDR program goals
- Discussion, Q&A

CDR Team

Jean Slutsky
Chief Engagement and Dissemination Officer

Bridget Gaglio
Program Officer

Chris Gayer
Program Officer

Michelle Henton
Program Associate

Sarah Chew
Program Assistant

Bill Lawrence
Senior Program Officer

Portfolio Overview

Background

- Patients, caregivers, and clinicians need to be equipped with the best available information for making informed decisions
- Knowledge about how to optimally communicate and facilitate the effective use of evidence, information, and tools by patients, caregivers, and providers is lacking in many areas
- Strategies are needed to make existing patient-centered outcomes research information available to patients and providers and to make the dissemination and implementation of this knowledge feasible in various contexts

CDR Funding Objective

The CDR program seeks to fund…

- Comparative effectiveness research…
  - that involves the direct comparison of effective health communication and dissemination interventions or strategies that engage patients, caregivers, and providers
  - in the context of real-world clinical-care settings and situations
  - to enable patients and caregivers to make the best possible decisions in choosing among available options for care and treatment

CDR Funding Priorities

Focus on 3 three key areas:

1. **Communication strategies** to promote the use of health and healthcare CER evidence by patients, clinicians, and others
2. **Dissemination strategies** to promote the use of health and healthcare CER evidence by patients, clinicians, and others
3. **Explaining uncertain health and healthcare CER** evidence to patients, clinicians, and others

Communication Strategies Example

Amplifying the patient's voice: Person-centered versus Measurement-based approaches in Mental Health

- Compares two ways for patients and prescribers to engage in shared decision making around medication treatment appointments in community mental health clinics: **patient-centered care** vs. **measurement-based care**
- Examines differences in patient-centered outcomes depending upon an individual's experience with medication treatment, level of intervention use, and the severity of their mental illness
- Includes 3,000 Medicaid-enrolled adults with severe mental illness who receive medication at one of 14 community mental health centers across the state of Pennsylvania

Kim MacDonald-Wilson, ScD
UPMC Center for High-Value Health Care
Pittsburgh, Pennsylvania

Dissemination Strategies Example

Comparing Traditional and Participatory Dissemination of a Shared Decision-Making (SDM) Intervention

- Evaluates three alternative approaches to dissemination of an evidence based Asthma SDM Toolkit
- Aims to determine what dissemination strategy most effectively increases practice level adoption of shared decision making, improves patient outcomes, and increases patient involvement in care decisions
- Utilizes a partnership between a statewide Medicaid network and NCNC, a statewide consortium of research networks

Hazel Tapp, PhD, BSC
Carolinas Medical Center
Charlotte, North Carolina

Explaining Uncertainty Example

Describing the Comparative Effectiveness of Colorectal Cancer Screening Tests: The Impact of Quantitative Information

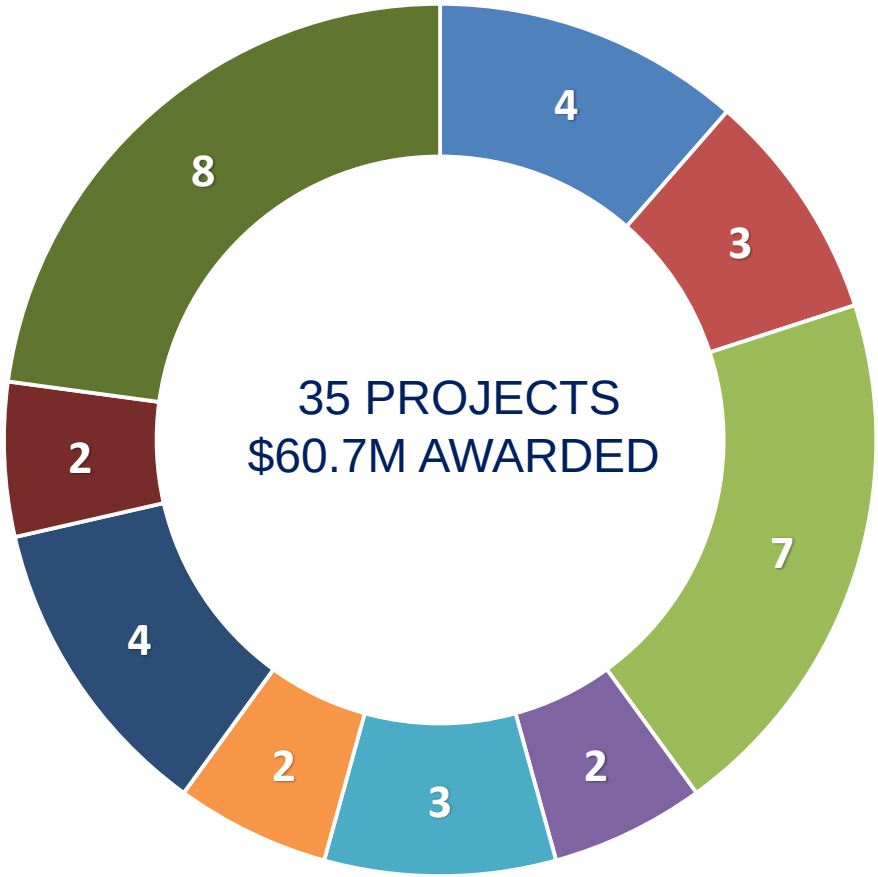
- Evaluates the impact of viewing quantitative information in a decision aid on screening intention, screening behavior and perceptions of risk in patients eligible for colorectal cancer screening
- Examines whether numeracy moderates the effect of quantitative information on screening behavior
- Uses a public deliberation exercise to review the results of the clinical trial and make recommendations about how decision aids should present quantitative information to patients

Peter Schwartz, MD, PhD

Indiana University

Indianapolis, Indiana

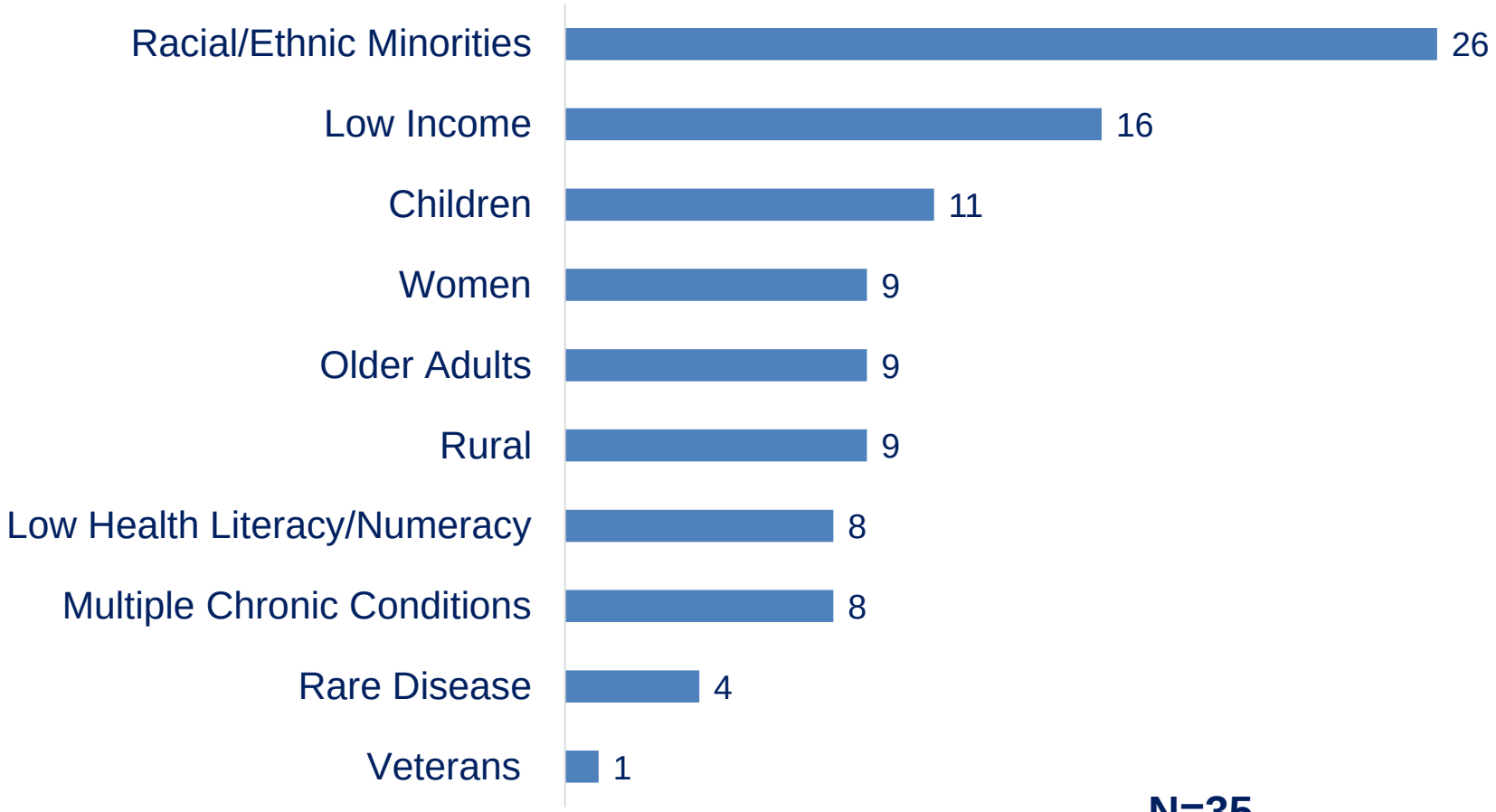
Portfolio by Disease/Condition



- Mental/Behavioral Health Disorders
- Cardiovascular Diseases
- Cancer
- Neurological Disorders
- Reproductive and Perinatal Health
- Kidney Diseases
- Multiple Chronic Conditions
- Respiratory Diseases
- Other*

* Other includes: Diabetes (1), CT Scan Radiation Dose (1), Rare Genetic Disorders (1), etc.

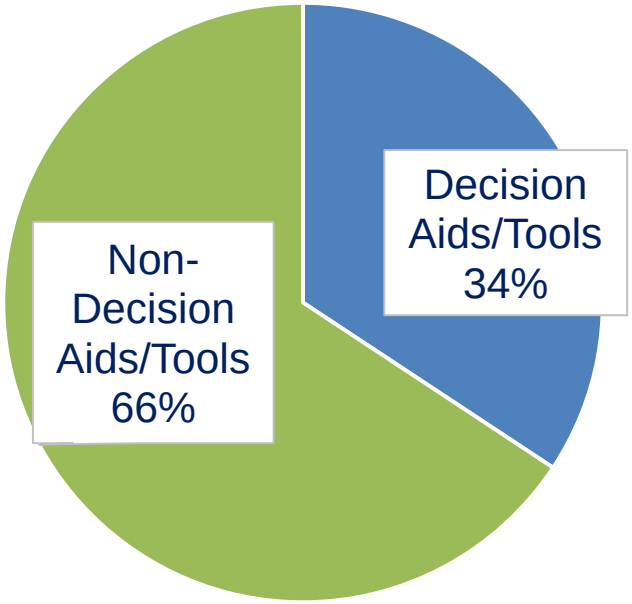
Portfolio by PCORI Priority Populations*



N=35

***Not mutually exclusive**

Decision Aids in CDR Portfolio



Tools that help patients understand:

- evidence about clinical management options
- their preferences about clinical outcomes
- so as to engage in shared decision making for making choices among those options

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- In early cycles, several CDR projects focused significant effort towards the development, validation, and pilot-testing of decision aids and tools
- The development, testing, and validation of individual decision aids/tools is currently considered non-responsive to the CDR funding announcement
- Emphasis is on the comparisons of demonstrated interventions, strategies, and tools, including validated tools for shared decision making

Decision Aid Work Group

- Based on the large number of decision aids funded across PCORI, CDR created a science-wide work group to examine the decision aid portfolio
- Conduct an extensive and exhaustive search to identify, categorize, and describe PCORI's decision aid/tool portfolio
- Findings will inform:
  - Strategic portfolio development
  - Future funding announcements

CDR 2015 Program Goals

- Launch the CDR Advisory Panel
- Refine the CDR Broad PFA
- Consider larger, more targeted investments
  - Host/Manage a CDR relevant Pragmatic Clinical Study
  - Explore opportunities for priority topics/targeted funding especially around dissemination of robust CER findings
- Contribute to PCORI Dissemination and Implementation Framework
  - Develop PCORI conceptual dissemination framework
  - Develop limited competition funding announcement among PCORI awardees for disseminating PCORI research results
  - Create the infrastructure to translate and disseminate robust CER findings for different audiences in coordination with AHRQ

Discussion, Q&A

Identifying Topics for Targeted Funding Announcements and Pragmatic Clinical Studies- Roles of SOC, Staff and Advisory Panels

Christine Goertz, DC, PhD
Chair, Selection Committee

Joe Selby, MD MPH
Executive Director

Current Status of Topic Generation and Prioritization at PCORI

- PCORI has solicited, received and prioritized topics for nearly two years
- Our processes have included:
  - Initial review of submitted topics by staff
  - Preparation of topic briefs usually by outside research teams;
  - Review and prioritization by PCORI's Multi-stakeholder Advisory Panels;
  - Review and approval by the SOC;
  - Multi-stakeholder workshops on selected topics
  - Development of targeted PFAs for selected topics or placement of topics on Pragmatic Clinical Studies High Priority List
- Until now, SOC involvement has come relatively late in the process – after the Advisory Panel Review – and earlier stages of the process have not usually been discussed with the SOC
- Until now, process has not been highly transparent to stakeholder communities

Pathway to a Funding Announcement

Pathway to a Funding Announcement

Staff use Tier 1 and Tier 2 review criteria to determine topic eligibility

↓

Science Oversight Committee (SOC) selects topics for topic briefs

↓

SOC reviews topic briefs

↓

Advisory panels use Tier 3 review criteria to prioritize research questions

↓

SOC selects topics for further development; workgroups refine research questions

↓

Staff and SOC use Tier 4 review criteria to assess research questions; SOC assigns research questions to targeted or Pragmatic Clinical Studies PFA

↓

Board reviews/approves research questions for targeted PFAs

SOC reviews and approves questions for Pragmatic Clinical Studies PFA

Pathway to a Funding Announcement

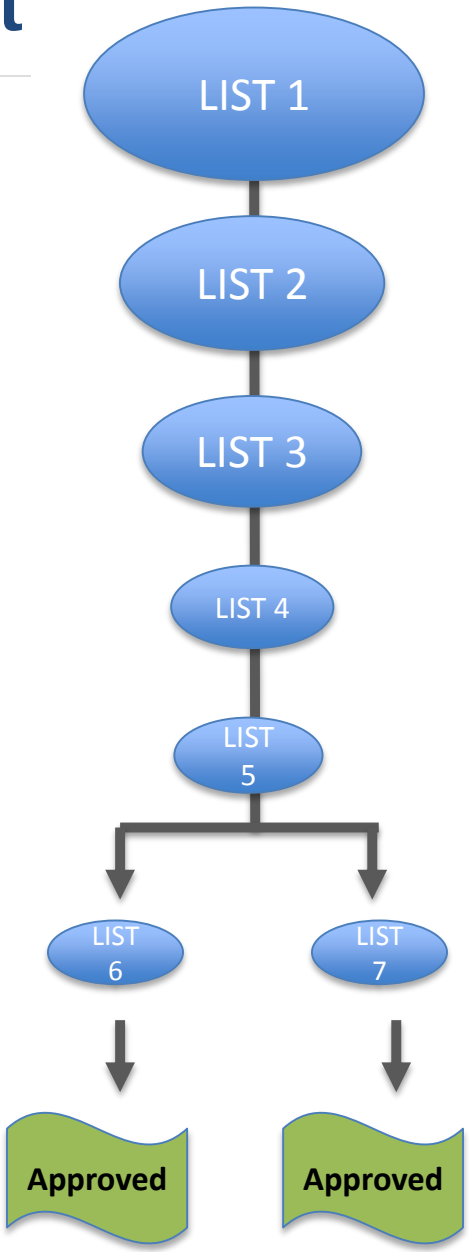
Staff use Tier 1 and Tier 2 review criteria to determine topic eligibility, producing List 1

↓

SOC selects topics for topic briefs, producing List 2

↓

SOC reviews topic briefs, producing List 3



Tier 1 Criteria: Determine Eligibility

- Is this a comparative effectiveness research question?
  - Are two or more options (one of which can be usual care) being compared? - **eligible**
  - Or is it instead a comment, a descriptive question, or a question of disease causation or biological mechanism **- ineligible**
  - Does the study involve cost comparisons or cost-effectiveness analyses **- ineligible**
  - Does the study fit at least one of PCORI's National Priorities for research **- eligible**
- Is this question duplicative with another question already in the research topic database? - **ineligible**
- Is the question patient-centered: i.e., is the comparison relevant to patients, their caregivers, clinicians, or other key stakeholders, and are the outcomes relevant to patients? - **eligible**

Tier 2 Criteria: Screening by Program Staff

- Large impact (burden) of the condition on the health of individuals and populations
- Important evidence gap is believed to exist (e.g. by virtue of a recent, credible evidence synthesis or clinical guideline)*
- Could PCORI-funded research close or help to close this evidence gap?
- How likely would implementation of relevant findings into practice be? (e.g., do one or more major stakeholder groups endorse the question)*
- Lack of evidence of other current/potential funders of this research*

* Based on initial consultations with patient, clinician or other stakeholders, researchers, or funding agencies

Pathway to a Funding Announcement

Staff use Tier 1 and Tier 2 review criteria to determine topic eligibility, producing List 1

↓

SOC selects topics for topic briefs, producing List 2

↓

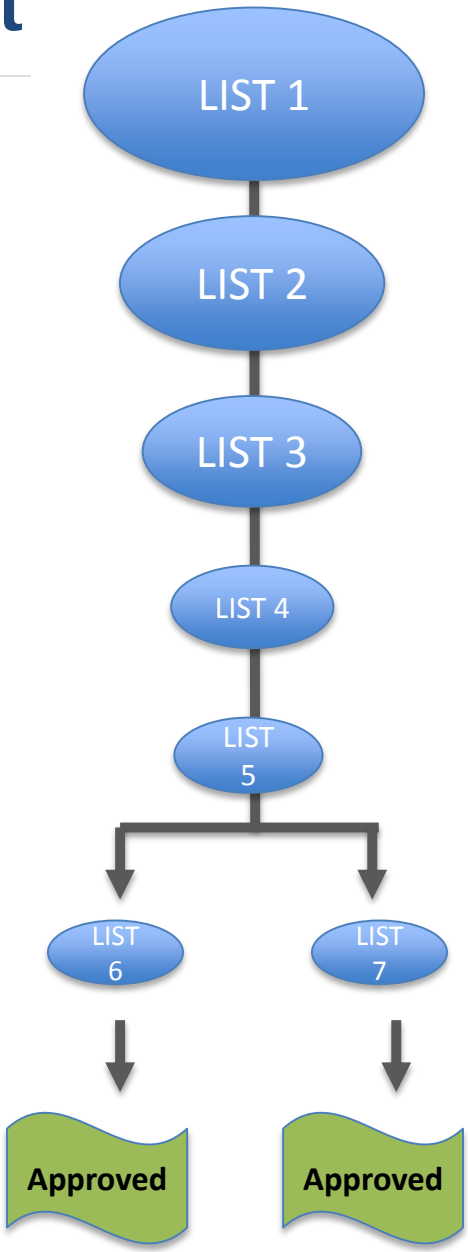
SOC reviews topic briefs, producing List 3

↓

Advisory panels use Tier 3 review criteria to prioritize research questions, producing List 4

↓

SOC selects topics for further development; workgroups refine questions, producing List 5



Tier 3 Criteria: Advisory Panel Criteria

(Applied by Advisory Panels (APs) after reviewing topic briefs)

- **Patient-Centeredness:** Is the comparison relevant to patients, their caregivers, clinicians or other key stakeholders and are the outcomes relevant to patients?
- **Impact (Burden) of the Condition on the Health of Individuals and Populations:** Is the condition or disease associated with a significant burden in the US population, in terms of disease prevalence, costs to society, loss of productivity or individual suffering?
- **Ongoing Evidence Gap:** Does the topic reflect an important evidence gap related to current options that is not being addressed by ongoing research
- **Likelihood of Implementation in Practice:** Would new information generated by research be likely to have an impact in practice? (e.g. do one or more major stakeholder groups endorse the question?)
- **Durability of Information:** Would new information on this topic remain current for several years, or would it be rendered obsolete quickly by new technologies or subsequent studies?

Pathway to a Funding Announcement

Staff use Tier 1 and Tier 2 review criteria to determine topic eligibility, producing List 1

↓

SOC selects topics for topic briefs, producing List 2

↓

SOC reviews topic briefs, producing List 3

↓

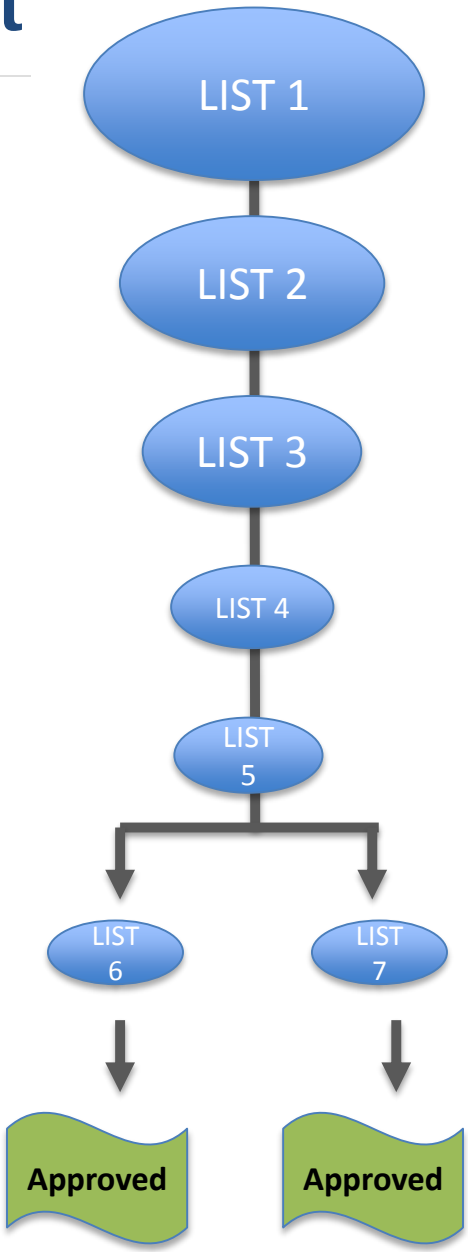
Advisory panels use Tier 3 review criteria to prioritize research questions, producing List 4

↓

SOC selects topics for further development; workgroups refine questions, producing List 5

↓

Staff and SOC use Tier 4 review criteria to assess questions; SOC assigns questions to targeted or Pragmatic Clinical Studies PFA, producing Lists 6 and 7



Tier 4 Criteria: Targeted PFA Criteria

(Distinguishing topics for targeted PFAs from topics for Pragmatic Clinical Studies list)

- **A specific question (or questions)** has been identified about prevention, diagnostic, or treatment options or system-level interventions that are currently covered and in use in at least some settings*
- The **importance of the topic** as determined by high scores from the advisory panel, strong interest from one or preferably more than one key stakeholder groups, and strong assessment of potential to change practice, warrants set aside funding and closer involvement in the study by PCORI
- **May require higher level of funding than the usual pragmatic clinical study** – either for larger sample size, longer follow-up or more complex interventions/data collection needed to pursue the specific question

*May require a multi-stakeholder workgroup or other information collection before decision is made by SOC

Pathway to a Funding Announcement

Staff use Tier 1 and Tier 2 review criteria to determine topic eligibility, producing List 1

↓

SOC selects topics for topic briefs, producing List 2

↓

SOC reviews topic briefs, producing List 3

↓

Advisory panels use Tier 3 review criteria to prioritize research questions, producing List 4

↓

SOC selects topics for further development; workgroups refine questions, producing List 5

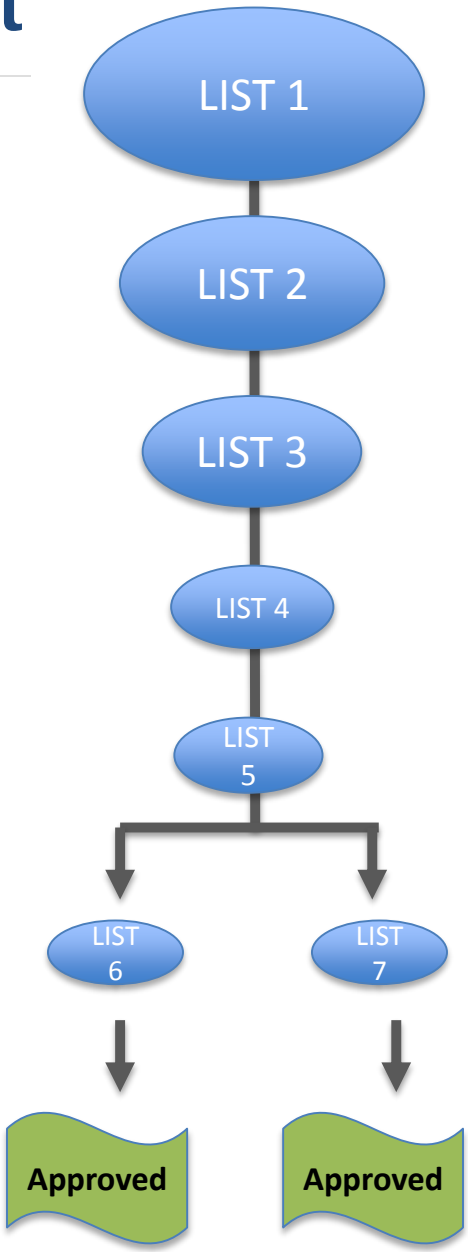
↓

Staff and SOC use Tier 4 review criteria to assess questions; SOC assigns questions to targeted or Pragmatic Clinical Studies PFA, producing Lists 6 and 7

↓

Board reviews and approves questions for targeted PFA

SOC reviews and approves questions for Pragmatic Clinical Studies PFA



Public Comment

Wrap Up and Adjournment

Grayson Norquist, MD, MSPH
Chair, Board of Directors

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