Board of Governors Meeting

Monday, December 8, 2014

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

Time	Agenda Item
10:15 a.m.	Welcome, Call to Order and Roll Call Consideration of November 18, 2014 Board Meeting Minutes for Approval
10:15- 10:20 a.m.	Reminder for Annual Update of Disclosures of Conflict of Interest Form
10:20- 11:00 a.m.	Executive Director's Reporter, End-of-Year Dashboard Review
11:00- 11:45 a.m.	Committee Reports
11:45- 12:15 p.m.	Public Comment
12:15- 1:00 p.m.	Break
1:00- 1:45 p.m.	PCORI Research Strategy Update
1:45- 2:15 p.m.	Development of a Targeted PCORI Funding Announcement on Hepatitis C
2:15- 2:45 p.m.	Release of PCORI Funding Announcement for PCORnet Phase II
2:45- 3:15 p.m.	Methodology Committee Update
3:15- 3:30 p.m.	Break
3:30- 4:15 p.m.	Engagement Update
4:15- 4:45 p.m.	Preliminary Results of WE-ENACT
4:45- 5:30 p.m.	Improving Methods for Conducting PCOR Program Overview
5:30- 5:45 p.m.	Public Comment
5:45 p.m.	Wrap Up and Adjournment

Board Vote: Approve the November 18, 2014 Board Meeting Minutes

Call for a Motion to:	• **Approve** November 18, 2014 Board Meeting Minutes
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

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Reminder for Annual Update of Disclosures of Conflict of Interest Form

Conflict of Interest Statements Due January 15, 2015

Lawrence Becker
Chair, Audit & Conflict of Interest Sub-committee

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Executive Director's Report

Joe Selby, MD, MPH
Executive Director

Patient-Centered Outcomes Research Institute

Transition

Vice-Chair, PCORI
Board of Governors
2010-2014

Vice-Chair, PCORI
Board of Governors
Beginning 2015

Brief Announcements

Review of Today's Agenda

End-of-Year Dashboard

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First GAO 5-Year Review of PCORI Is Nearing Completion

- GAO Review began in March. GAO identified two primary questions for the review:
  - To what extent has PCORI established research priorities and funded research in accordance with its legislative requirements?
  - To what extent has PCORI established plans and undertaken efforts to evaluate the effectiveness of its work?
- Entrance Conference with the GAO Team in April
- Meetings with GAO team in May, June, July, September to discuss Advisory Panels, Dissemination, Evaluation, Merit Review, PCORnet, Research Funding
- We expect to have our Exit Conference with GAO team in December; opportunity to review and comment on the GAO's draft report in mid-winter
- GAO's report to Congress is due in March of 2015

PCORI Policy on Peer Review of Primary Research and Public Release of Research Findings

- Public comment period for Draft Policy closed on Nov 7th
- Review of 835 discrete comments representing 63 individuals and organizations underway -- by AIR
- NIH-HHS Notice of Proposed Rulemaking on Reporting of Clinical Trials Results released on Nov 19th
- Revised Policy due to Board of Governors, via EDIC, on February 24, 2015

PCORI Dissemination and Implementation Framework and Toolkit Workshop – Dec 10

Goals of the Framework and Toolkit:

- Identify best practices and what they mean for how PCORI conducts dissemination of research findings
- Develop tools for planning D&I activities based on best practices
- Identify innovative ways to disseminate evidence, work with partners, and address D&I challenges

Purpose of the Workshop/Webinar

- Elicit diverse stakeholder and expert feedback on the Framework and Toolkit from representatives
- Identify new ways PCORI can partner with other organizations in D&I activities

Logistics

- Wednesday, December 10, 2014, 11:00 AM – 4:00 PM ET
- At Mathematica Policy Research, or via live webcast

PCORI's Inaugural Annual Meeting

- October 6-8, 2015, Washington, DC
- Highlighting early study results, high priority work-in-progress, marking our 5th anniversary
- Half-day of joint programming with AHRQ, focusing on dissemination and other collaborative initiatives
- One-and-a-half days of plenary sessions, breakouts, awardee workshops, "mini-summits" on major initiatives

Review of Today's Agenda

- End-of-Year reports from PCORI Committees
- Research Strategy Update from the SOC
- Targeted PFA Announcement – Hepatitis C
- PCORnet Phase II Funding Announcement
- Methodology Committee Update
- Update/Overview – PCORI's Engagement Program
- Evaluation Report – Early Data on Impact of Engagement
- Methods Research Program Overview

Comments or Questions?

Dashboard Review
End of FY 2014

Joe Selby, MD, MPH
Executive Director

Patient-Centered Outcomes Research Institute

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Board of Governors FY2014 Dashboard – Q4 (As of 9/30/2014)

Influencing Research

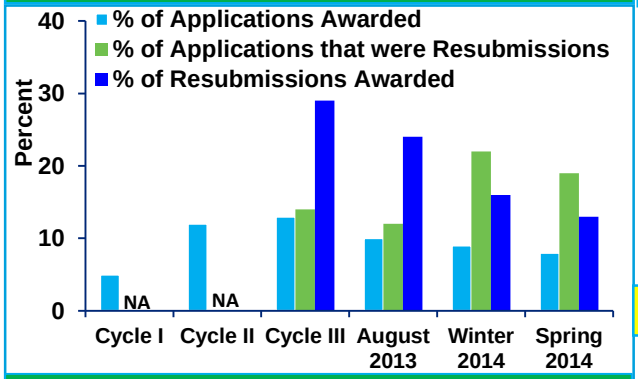
Working with PCORI influenced Geisinger Health System to incorporate patient engagement into its strategic plan for research and require it for all projects

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

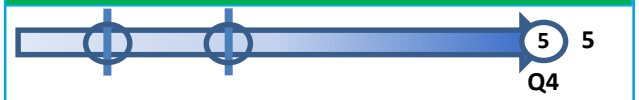
Legend

On Target | Off Target | Needs Attention | NA=Not Applicable | Target | Previous Actual | Current Actual

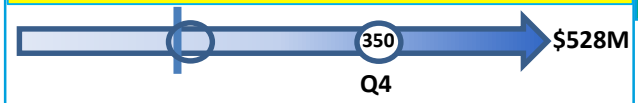
Success Rates for Broad Awards



Priority Topics to Be Awarded = 5



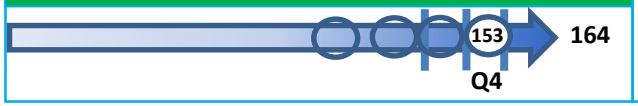
Funds to Be Committed to Research = $528M



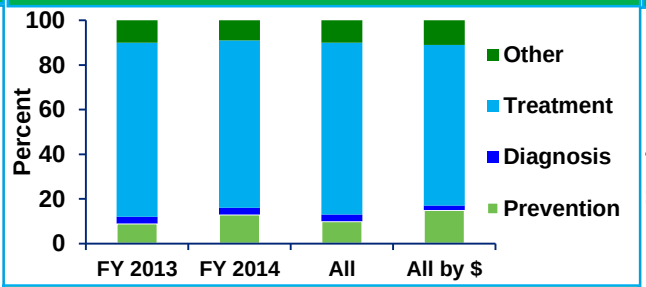
Funds to Be Expended on Research Awards=$94M

Other Expenditures Planned = $89M

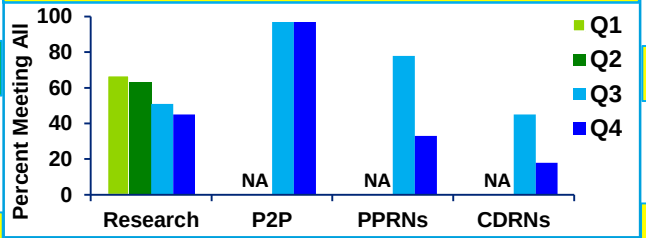
Planned Staffing Level = 164 People



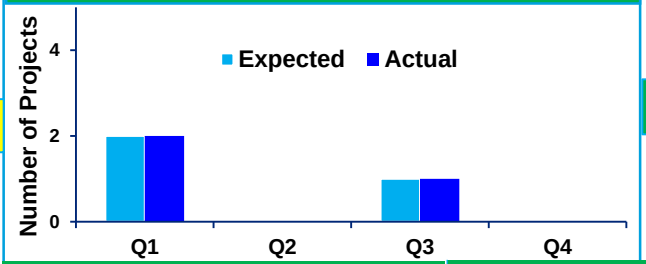
Composition of Funded Research Portfolio



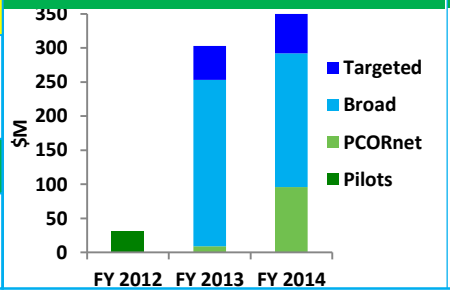
Percent of Projects Meeting All Milestones



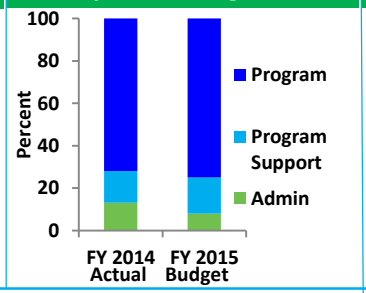
Completion of Research Projects



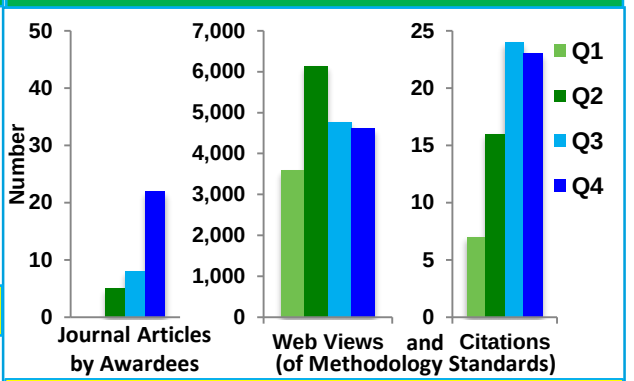
External Research Commitments



Expense Categories



Dissemination and Uptake Indicators



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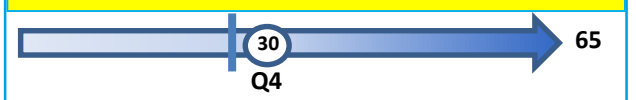
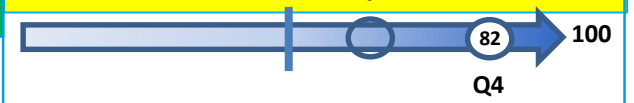
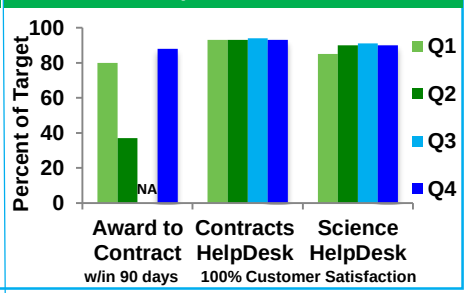


Ambassadors to Be Fully Trained = 100

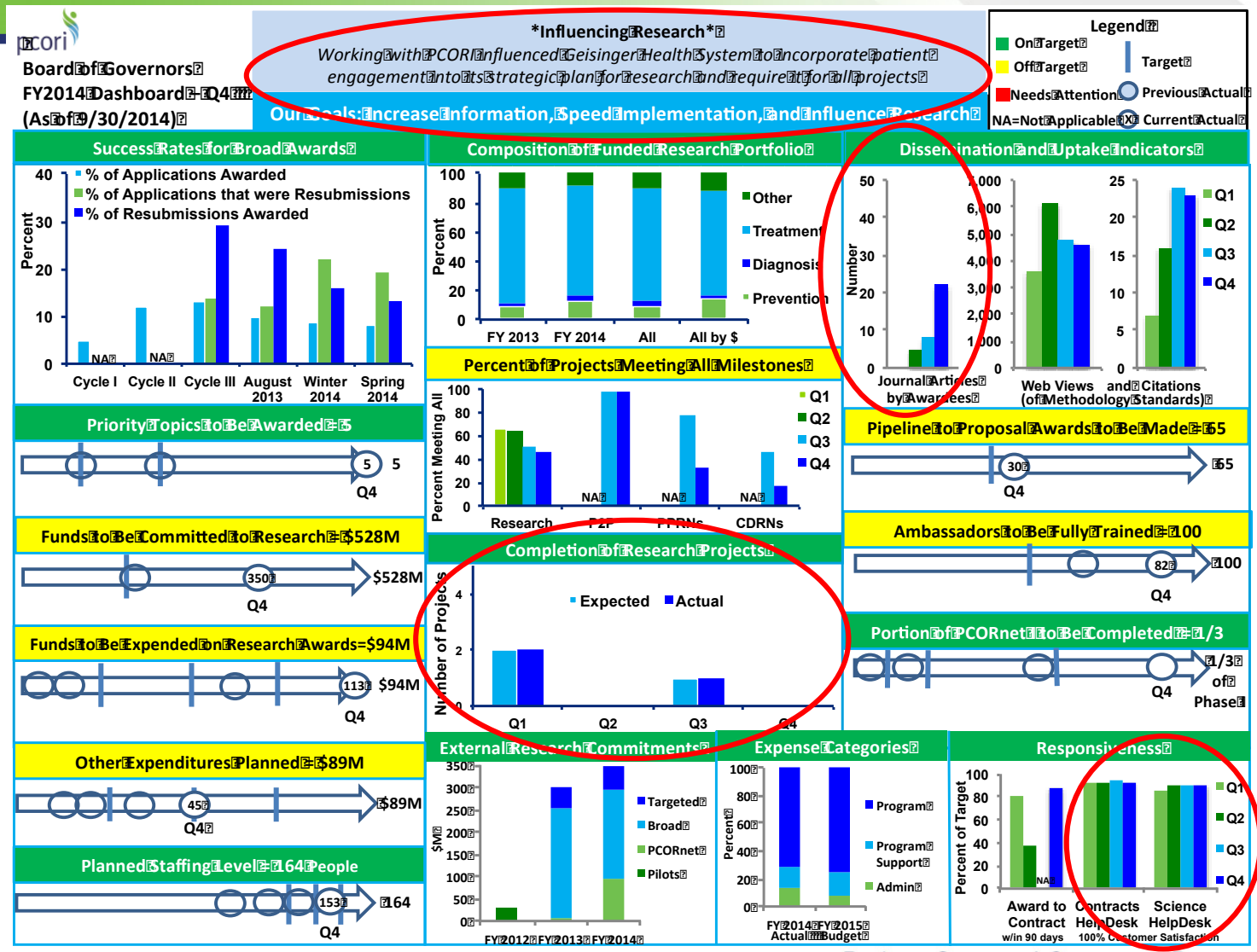


Portion of PCORnet I to Be Completed = 1/3

Responsiveness



New and Noteworthy Items



Goal 3: Influence Research

Early Signs

Influencing Research

Working with PCORI influenced Geisinger Health System to incorporate patient engagement into its strategic plan for research and require it for all projects

- Geisinger Health System (GHS) leadership met to evaluate their progress toward achieving goals in their 10 year research strategic plan. They discussed changes which had occurred in the research environment over those 6 years, to which they needed to adapt. They identified patient engagement in research as key area of emphasis.
  - GHS had received 2 PCORI awards over that period of time
- Patient Engagement Work Group was formed to explore the place of patient engagement in GHS research going forward
  - Key recommendation is that ***patient engagement be the default at GHS, not the exception***
  - Work Group's goal is to create an atmosphere conducive to engaging patients in GHS research by raising awareness and making resources available to GHS research community
  - Several PCORI engagement concepts were central to the work group's thinking and subsequently appeared in their final report and recommendation to leadership

GHS Patient Engagement Framework

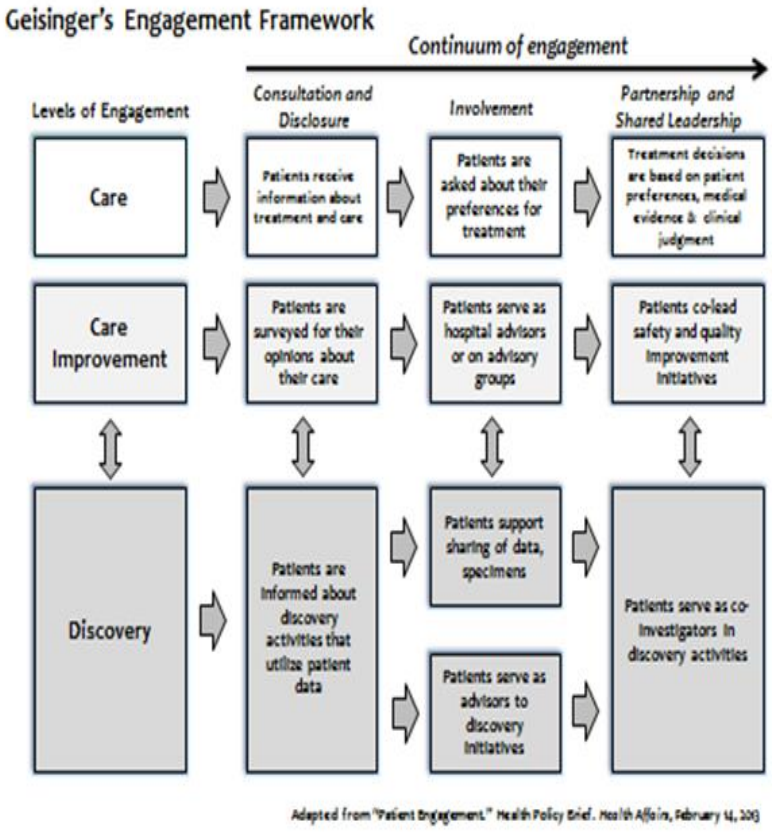
"..as we navigate our way toward enhancing patient engagement in research and discovery at Geisinger, PCORI has been and will continue to be both guide and anchor for that effort"*

GHS attributes their new focus on patient engagement in research to:

- Internal culture that has always valued a patient centered approach (readily adaptable)
- External forces, such as PCORI, which have advanced the national conversation around patient engagement and provided invaluable resources (both financial and intellectual) for making it possible

PCORI resources have served as a "lead impetus,"* and heavily informed GHS's conceptualization of their own patient engagement framework

Patient Engagement Goals have been built into GHS Strategic Plan and personnel goals of GHS leadership



*Dan Davis, PhD, Director, GHS Bioethics and Patient Engagement Work Group Lead

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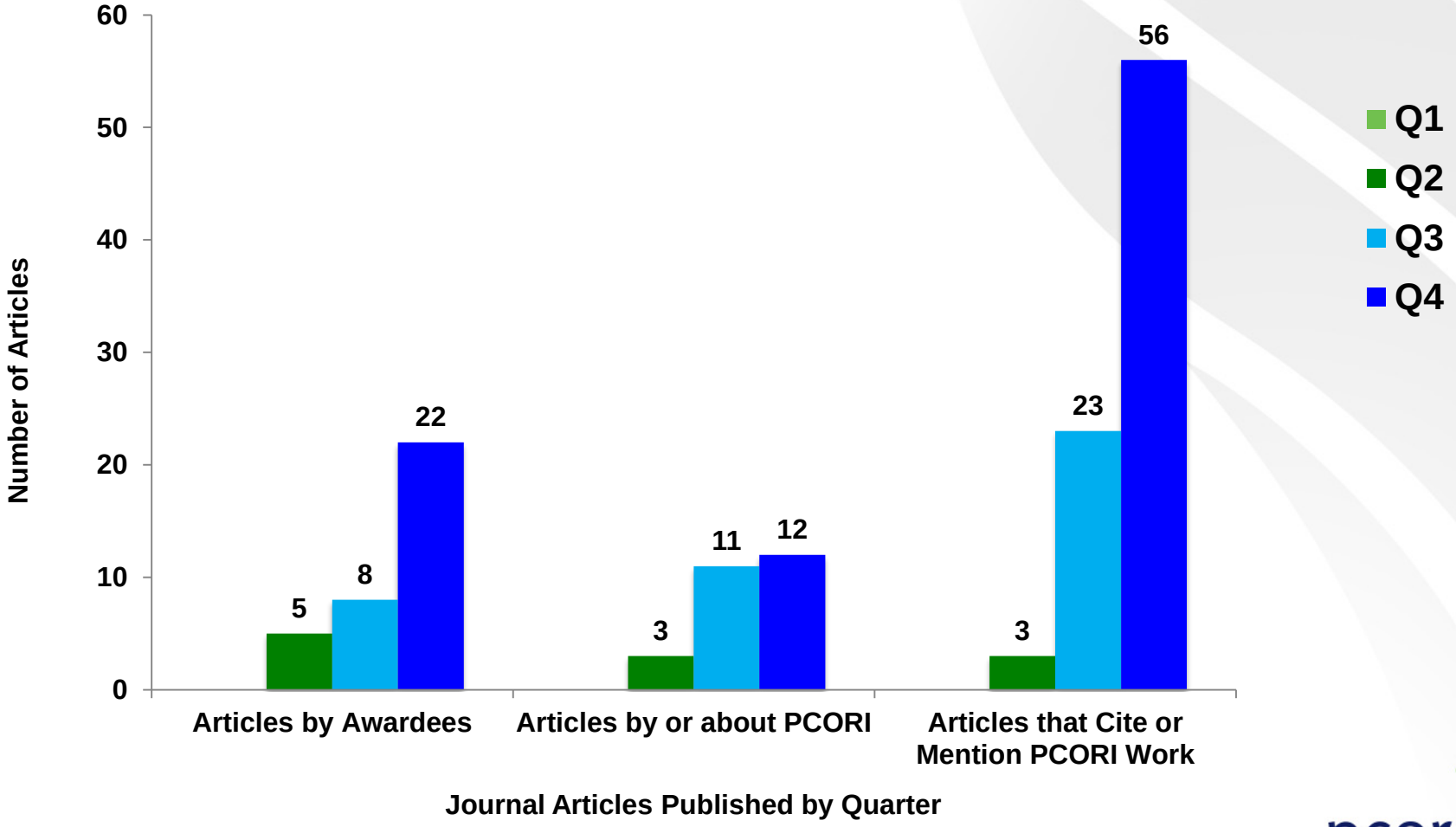
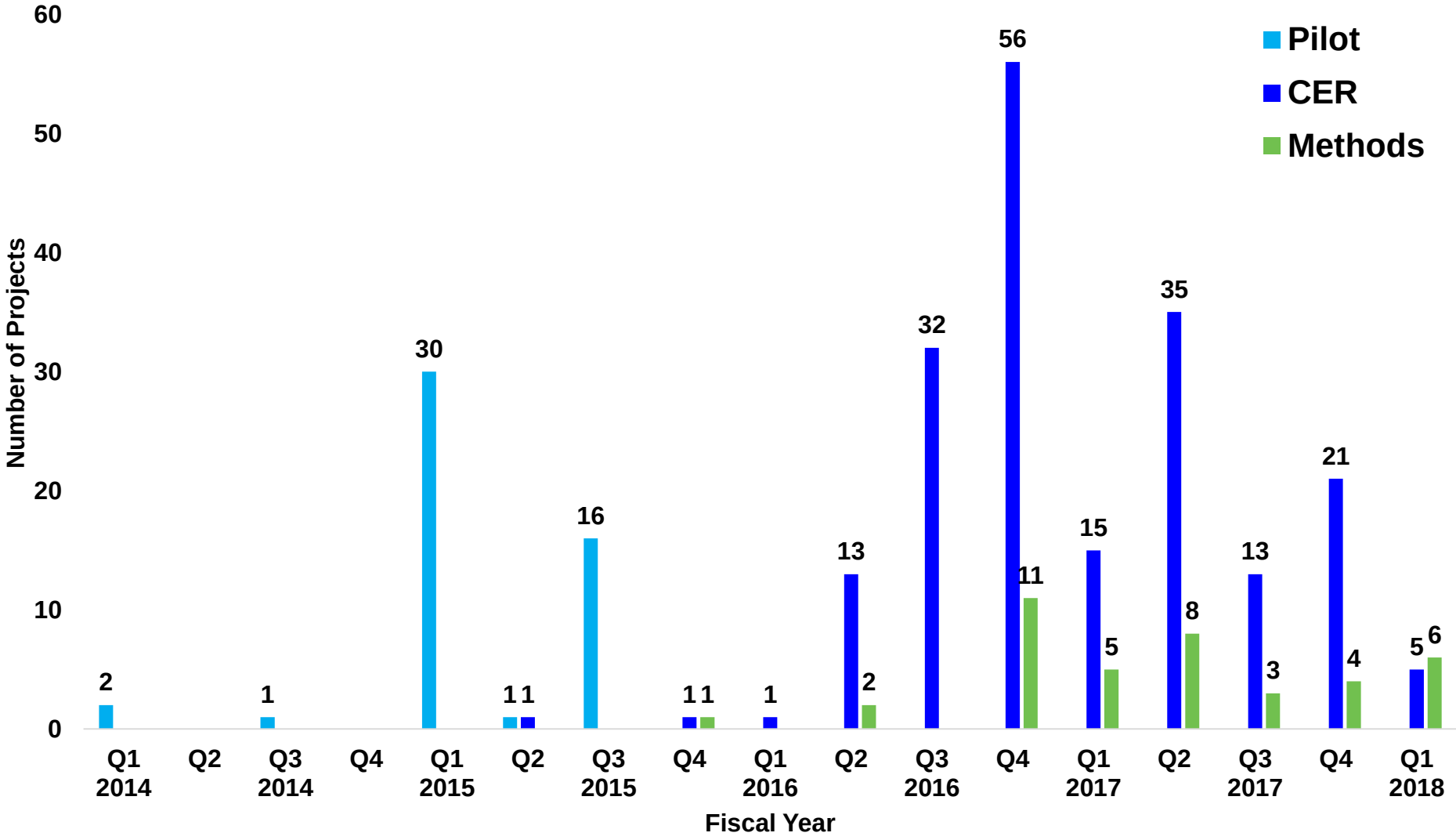
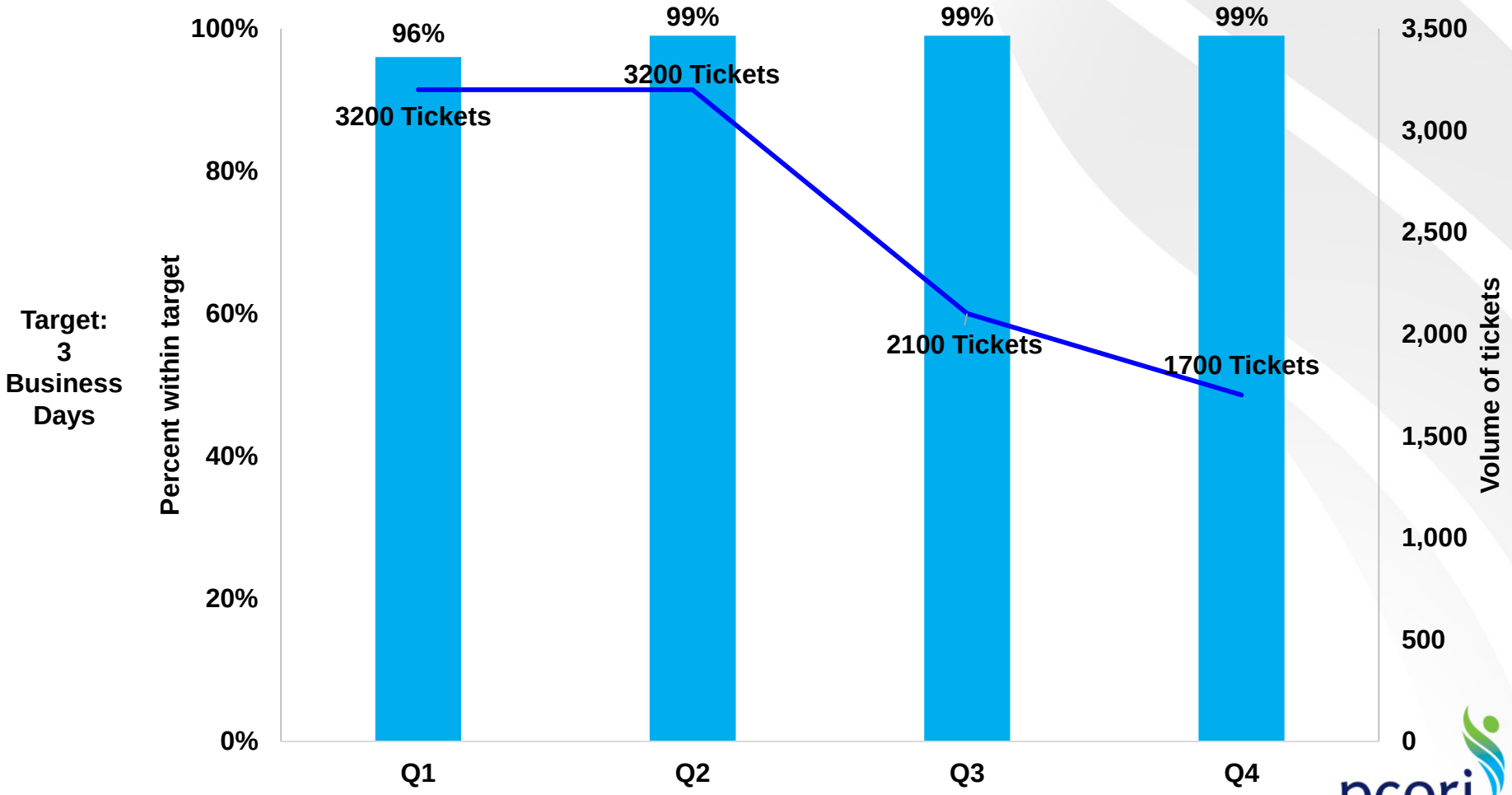
Number of Journal Articles Concerning PCORI



Expected Number of Contracts Ending per Quarter
(Includes estimates for some contract modifications in process)



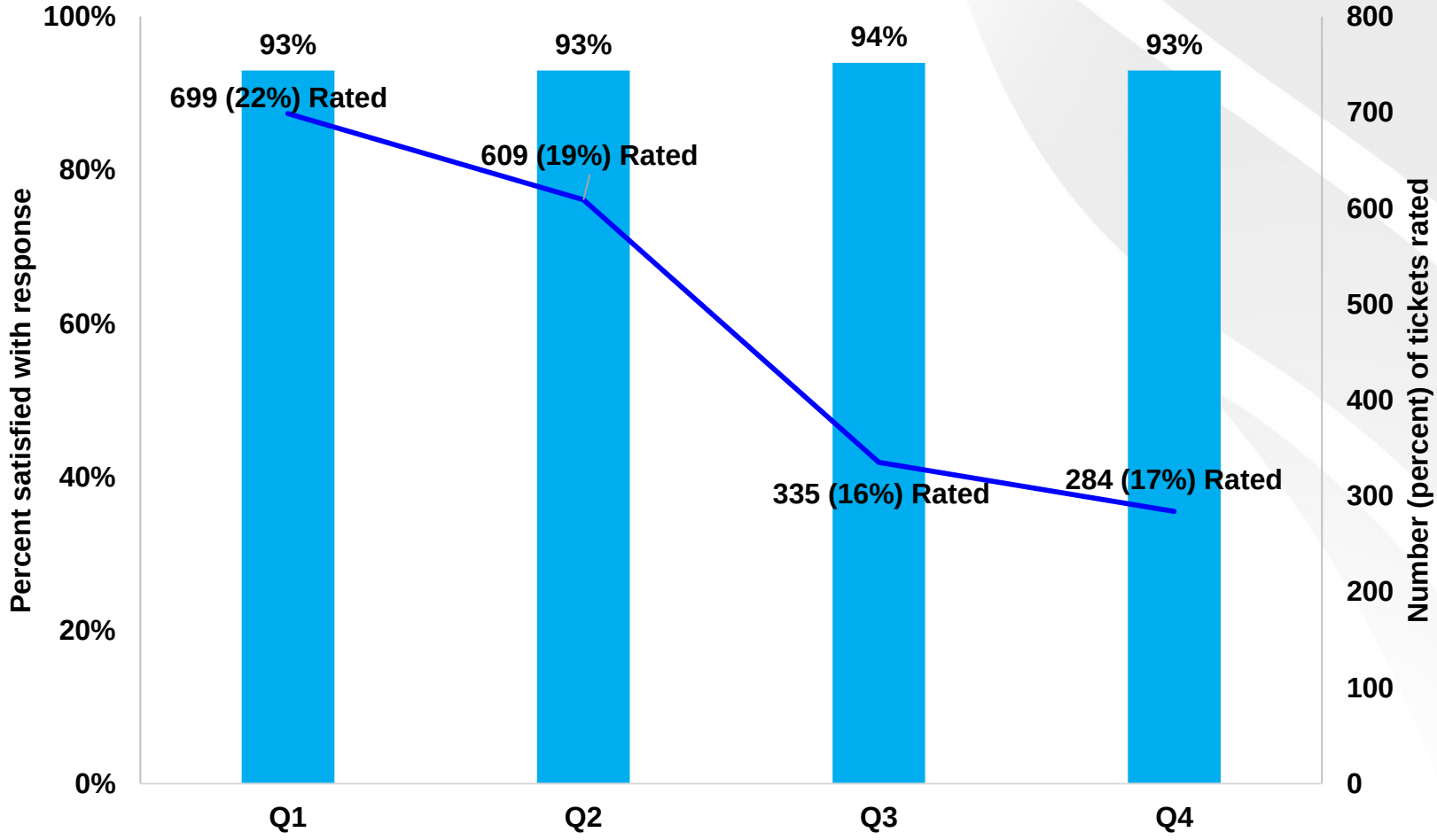
Contracts' Help Desk Response Time



Satisfaction with Contracts Help Desk Response

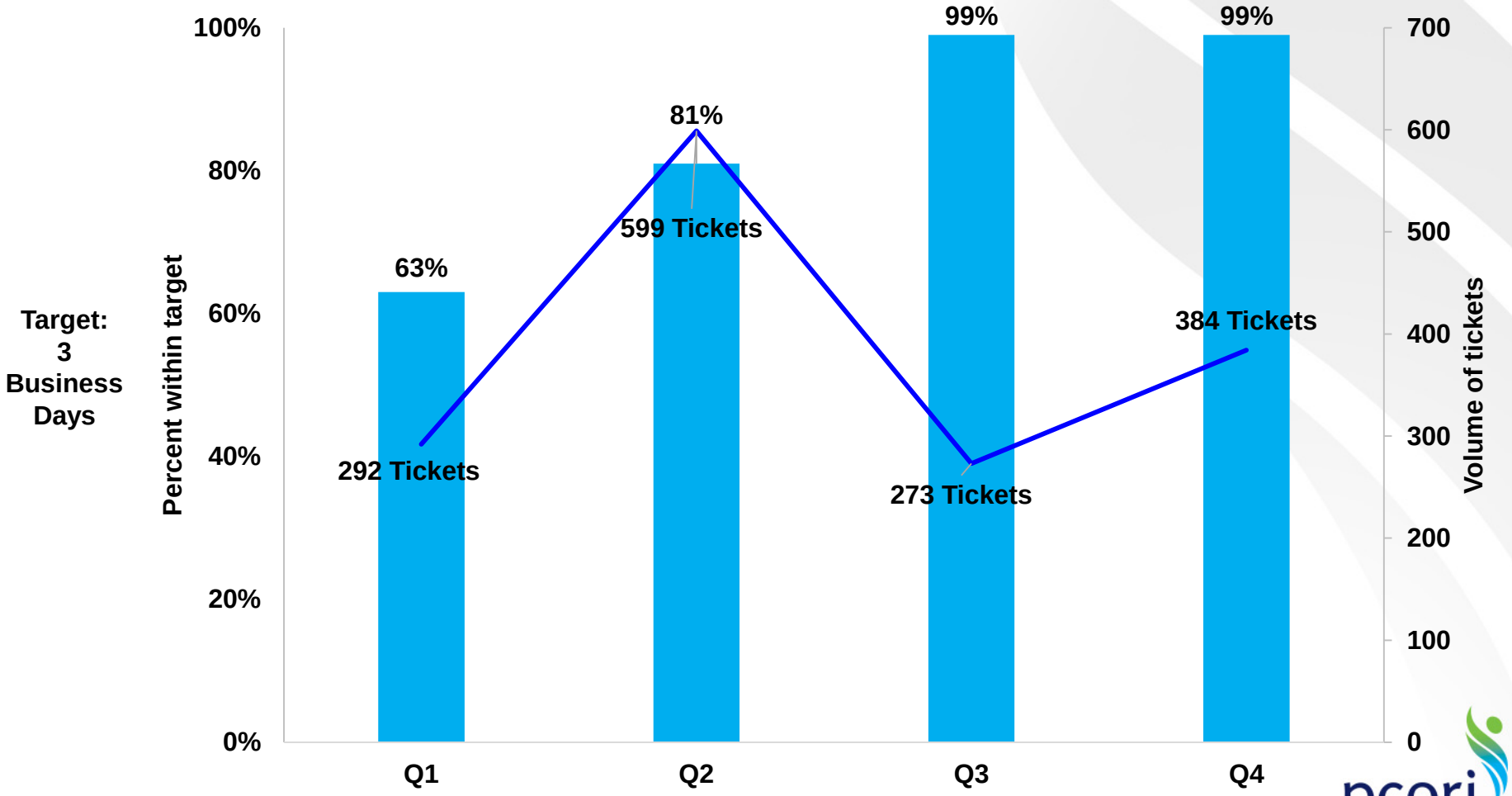
Data from software that handles help desk tickets

Staff follow-up with all respondents who indicated that they were dissatisfied



Industry average = 85% of respondents satisfied

Science Help Desk Response Time



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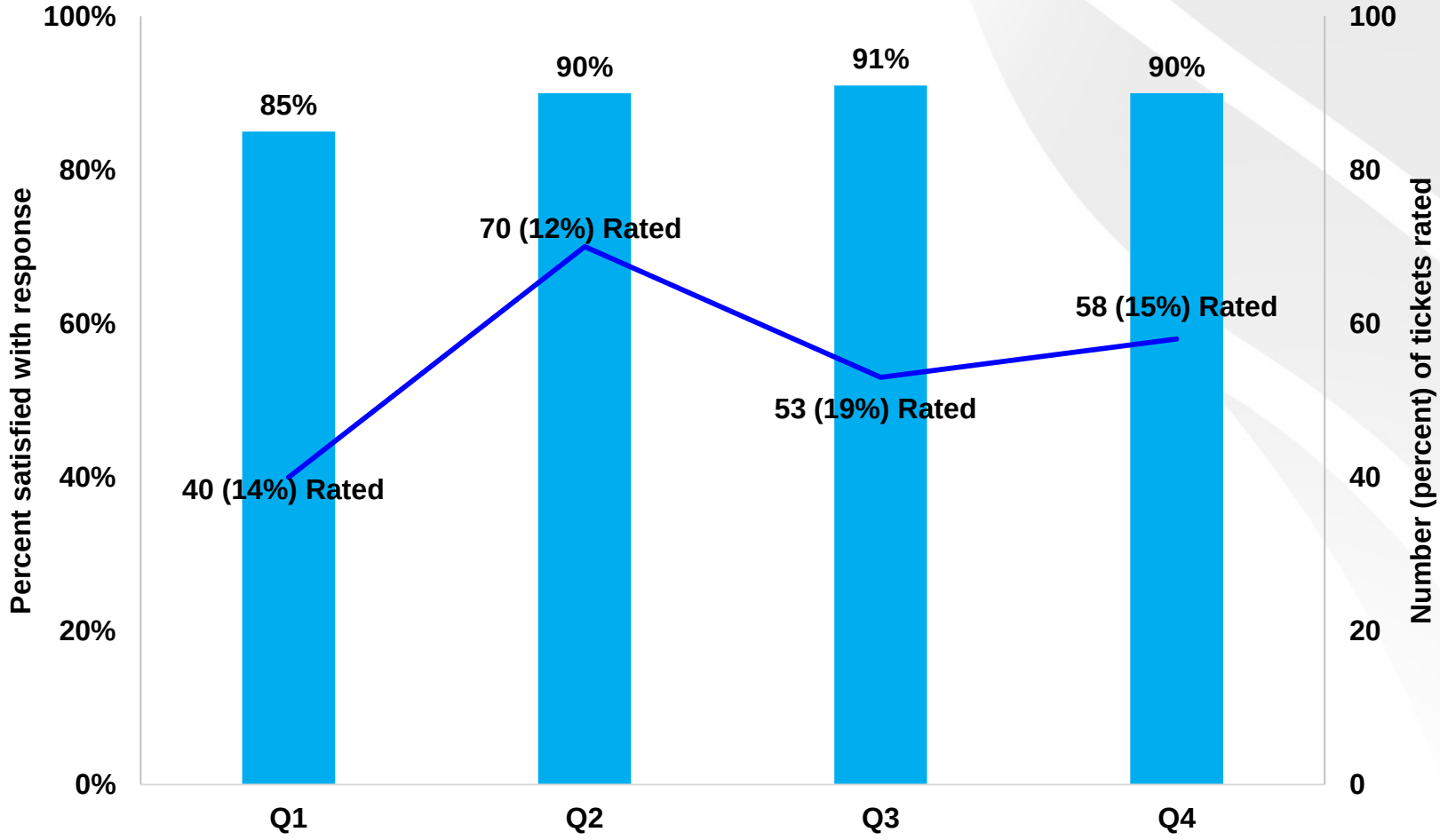

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Applicant Satisfaction with Support

Information about PCORI's Applicant Survey

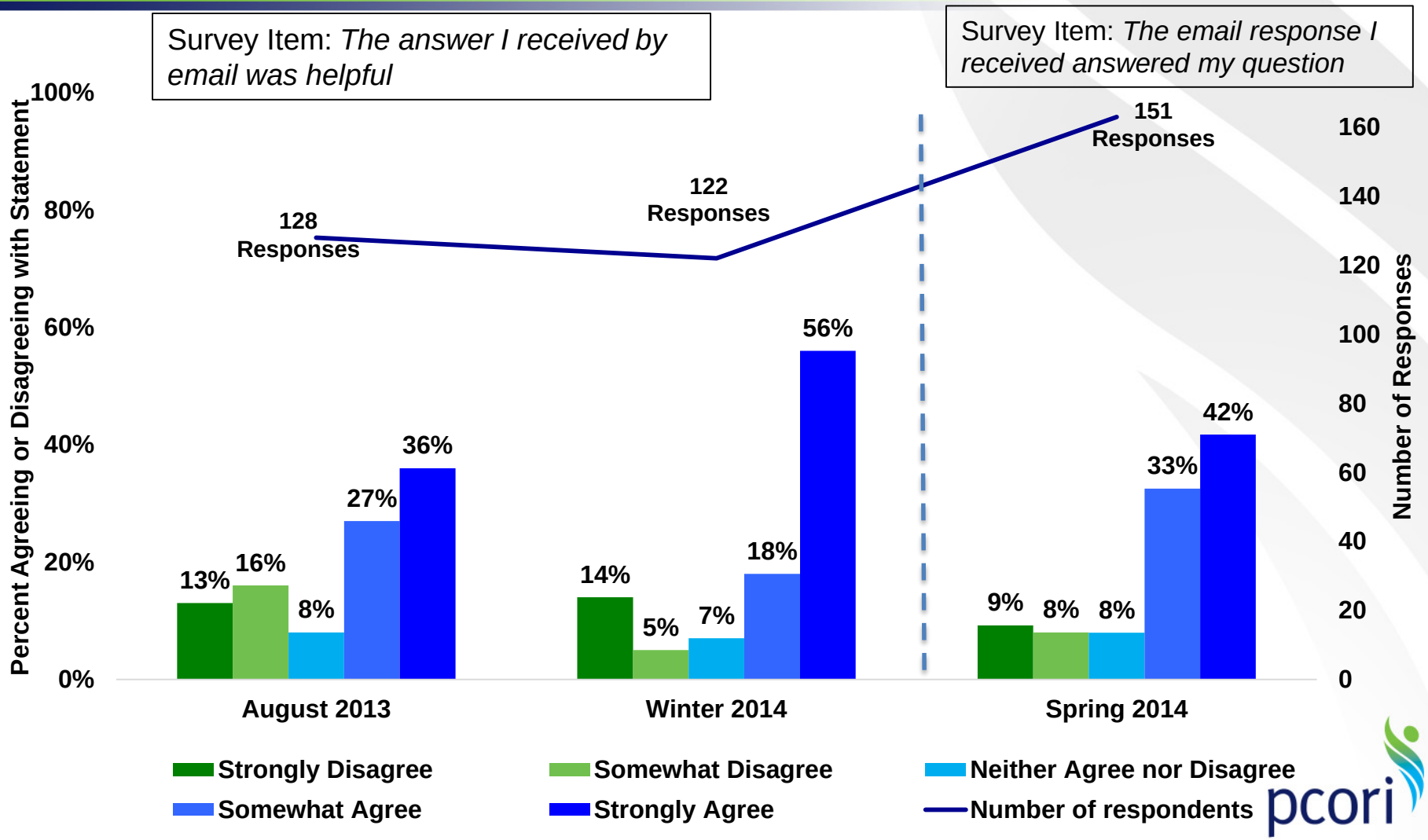
Completion Rates of Applicant Surveys *The survey is administered upon submission of application and is anonymous*			
Cycle	**August 2013**	**Winter 2014**	**Spring 2014**
Completion Rate	**27%**	**61%**	**43%**
Number of Respondents	**626**	**355**	**519**

Our Applicant Survey asks about the following sources of support for applicants:

- pfa@pcori.org – "Contracts"
- sciencequestions@pcori.org – "Science"
- Science Program Telephone Help Line

Ratings of Helpfulness of Email Support by Cycle

Data from PCORI's Applicant Survey (Contracts & Science combined)



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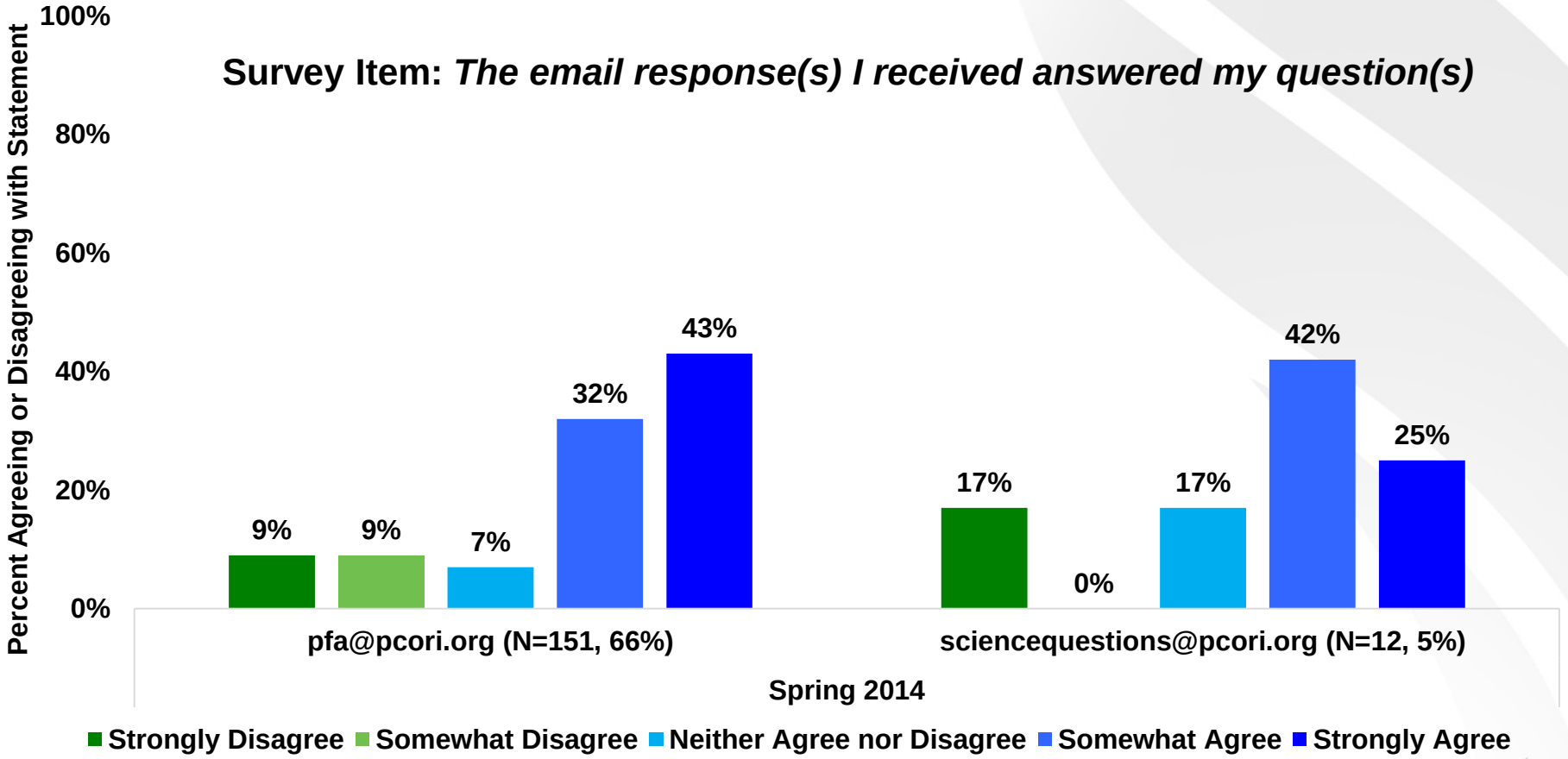
Ratings of Helpfulness of Email Support from Contracts Compared with that from Science for Spring Cycle (only)

(Note small number of respondents for Science)



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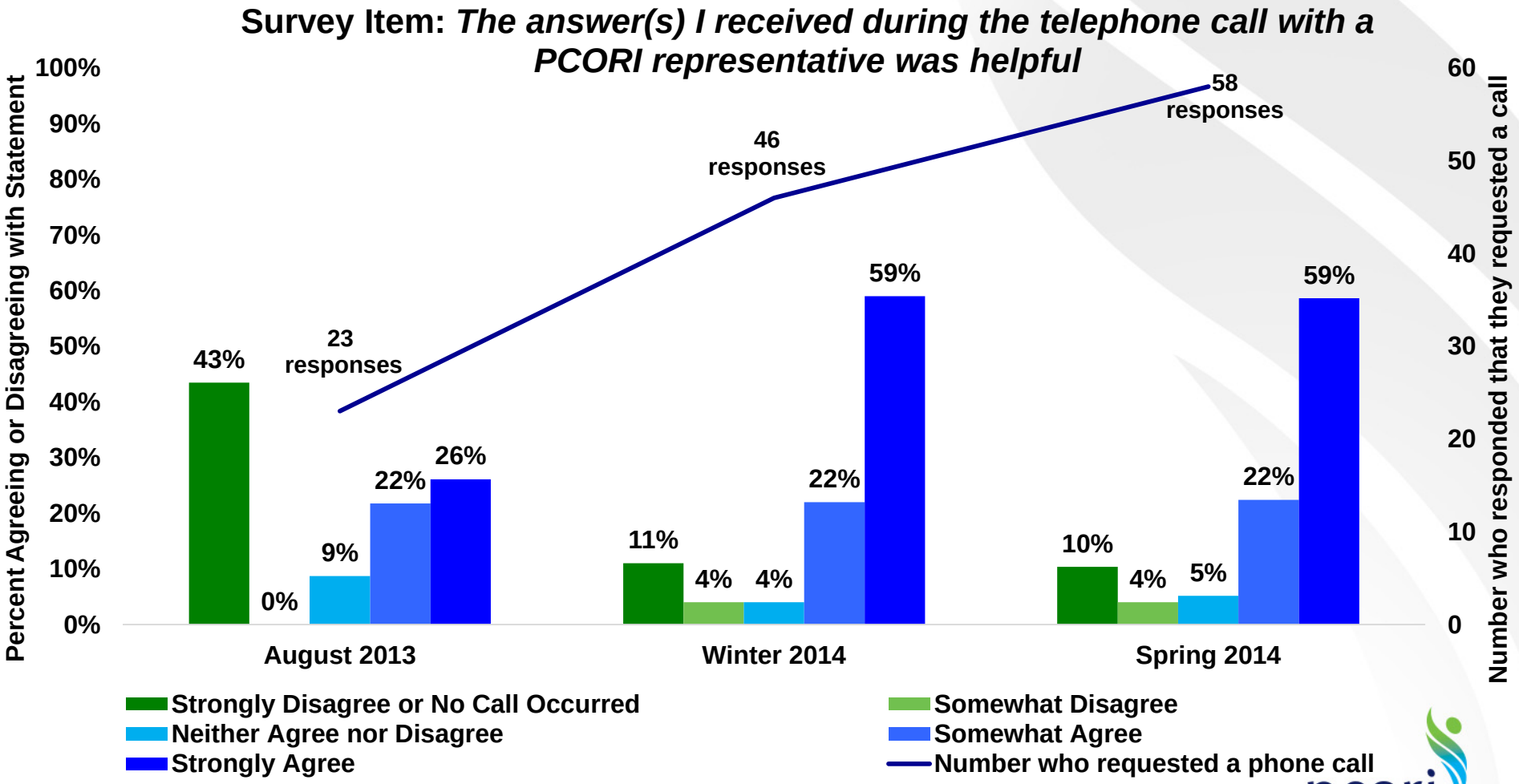
Ratings of Helpfulness of Telephone Support to Applicants Requesting a Phone Call by Cycle

Data from PCORI's Applicant Survey



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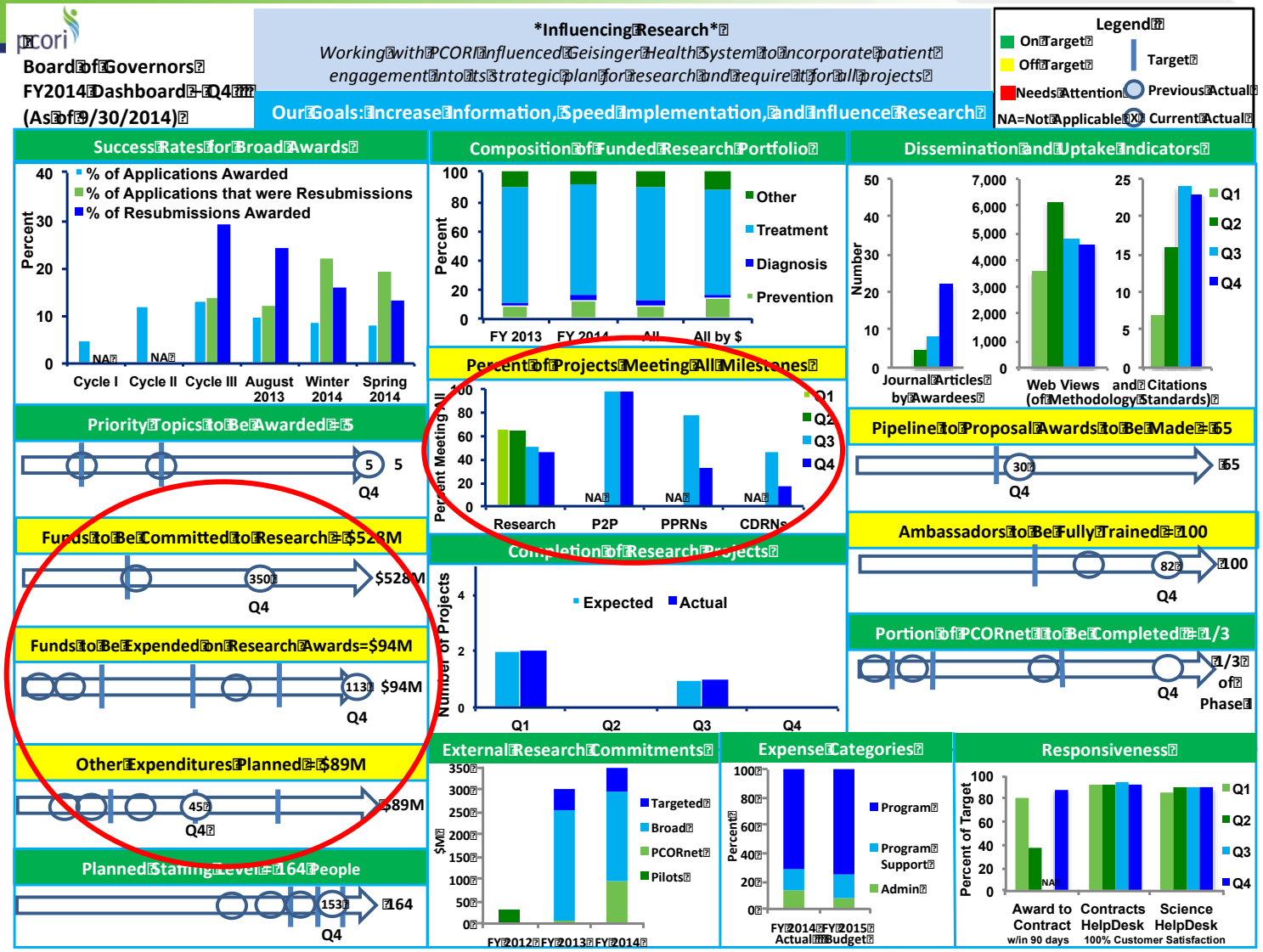
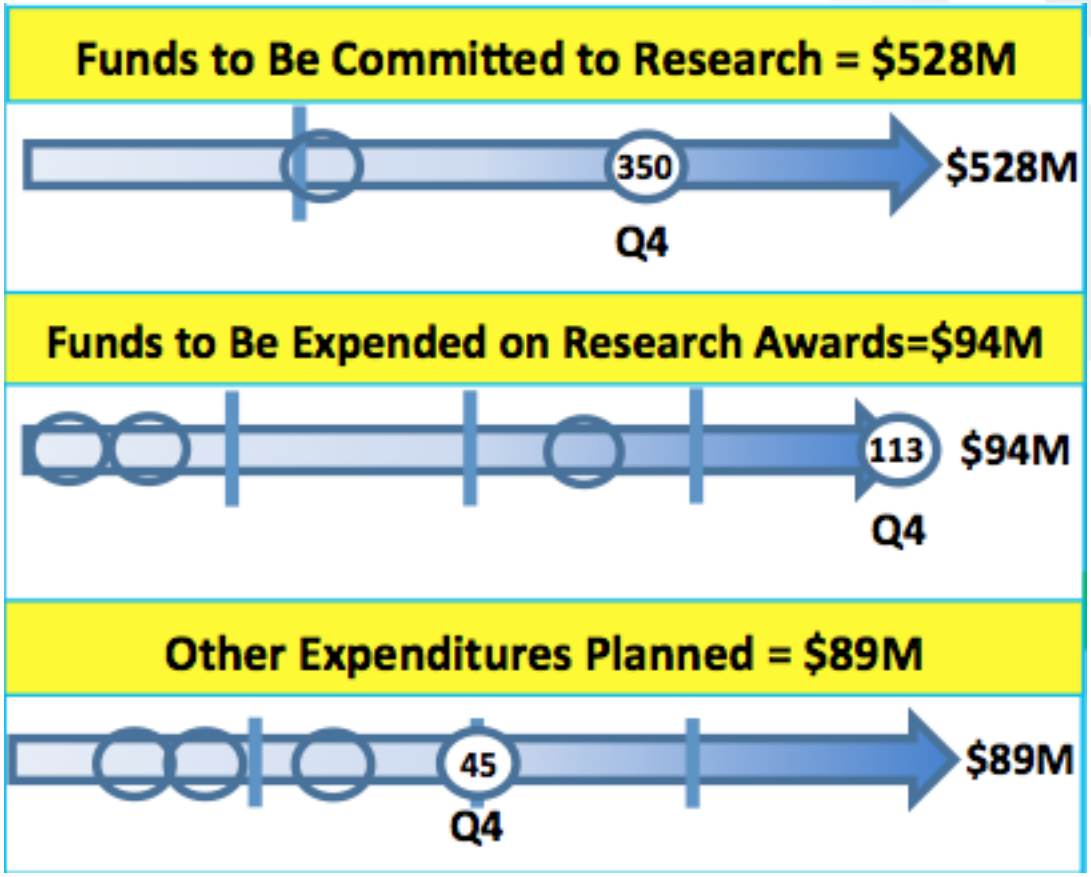
Yellow-Flagged Items – More than 10% Off Target



Commitments and Expenditures

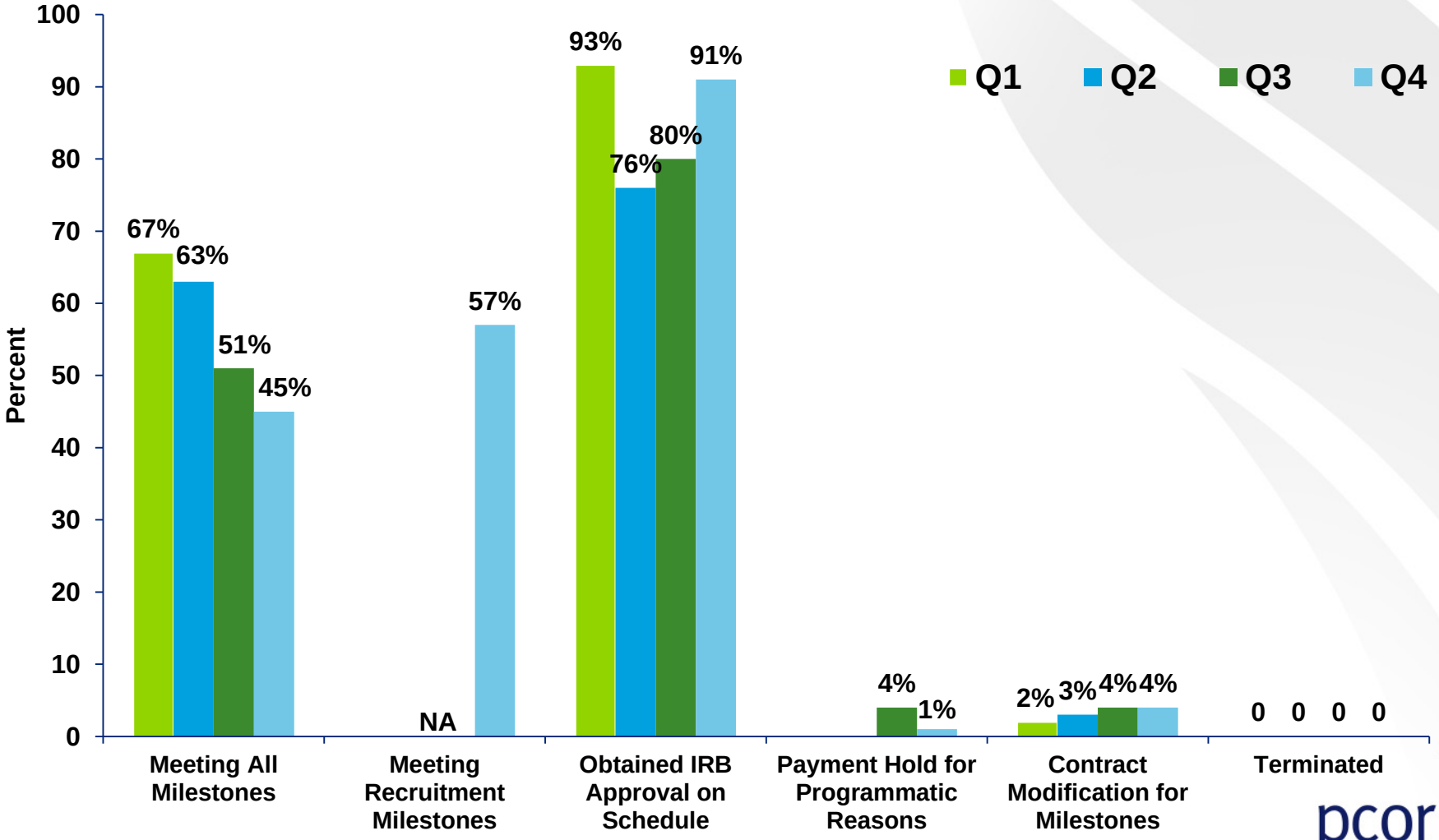


Progress of Research Projects – Milestones

	Q1	Q2	Q3	Q4
Number of Projects with a Progress Report Due • Predominantly 6-month report only • 80 with 12-month report also • A few with18-month report due next Q	23	57	140	186
Percent of Projects Meeting All Milestones Due	67%	63%	51%	45%
Average Percent of Milestones Due that Were Met	88%	87%	85%	84%
Percent of Projects Meeting Recruitment Milestones (47 projects with a recruitment milestone due by Q4 2014)	NA	NA	NA	57%

We continue to refine our approach to measuring and reporting on the progress of projects and will present additional analyses as we have sufficient data for them

Additional Measures of Progress of Projects



Discussion and Questions

- Are there new metrics suggested by today's presentation and discussion?
- Are there metrics that should be retired?

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Board of Governors FY2014 Dashboard – Q4 (As of 9/30/2014)

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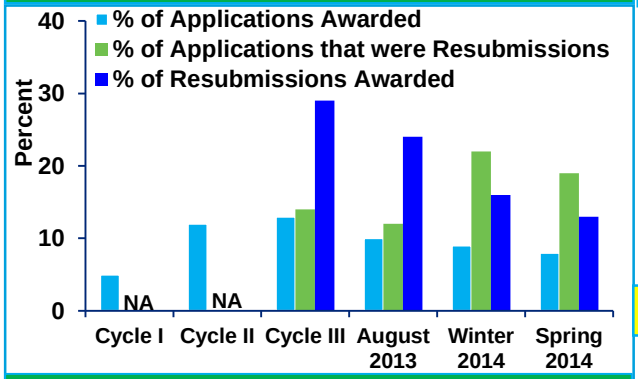

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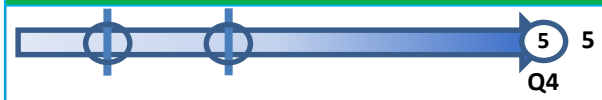

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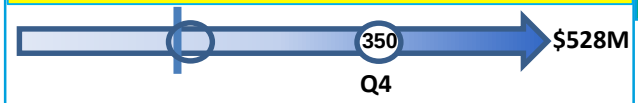

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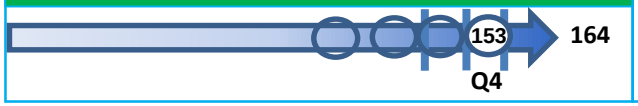
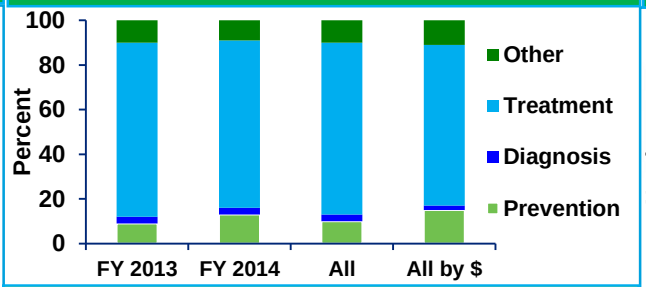
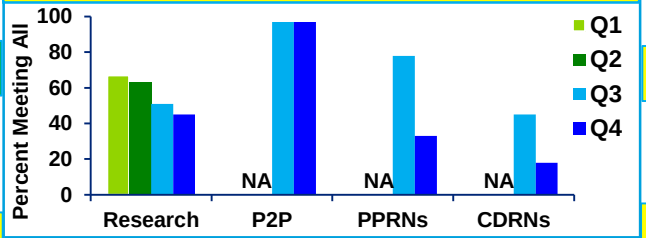


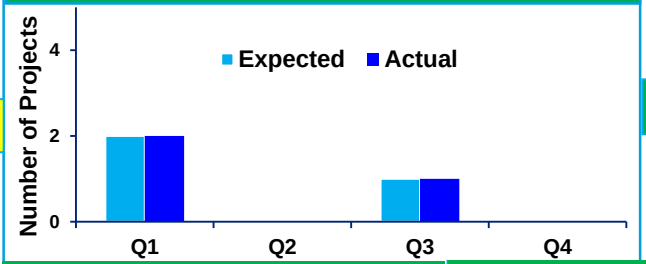
Composition of Funded Research Portfolio



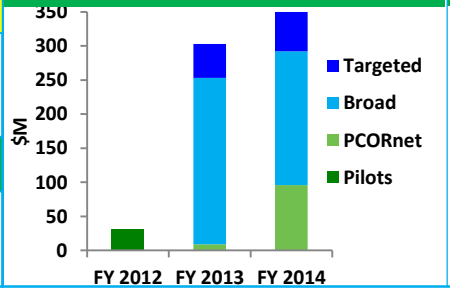
Percent of Projects Meeting All Milestones



Completion of Research Projects



External Research Commitments



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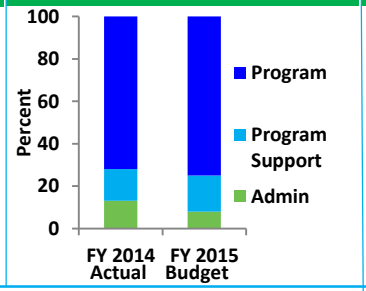
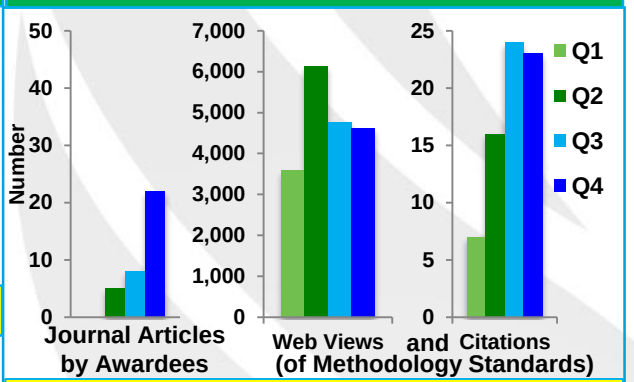


Dissemination and Uptake Indicators



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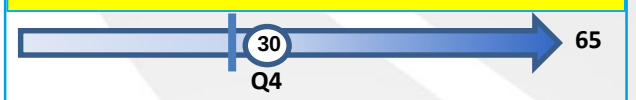
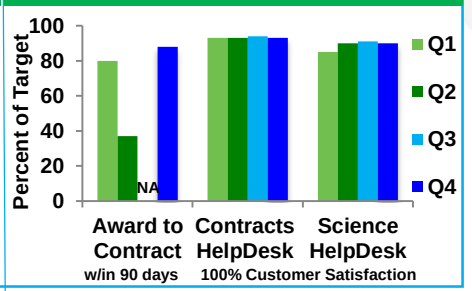


Ambassadors to Be Fully Trained = 100

Portion of PCORnet I to Be Completed = 1/3

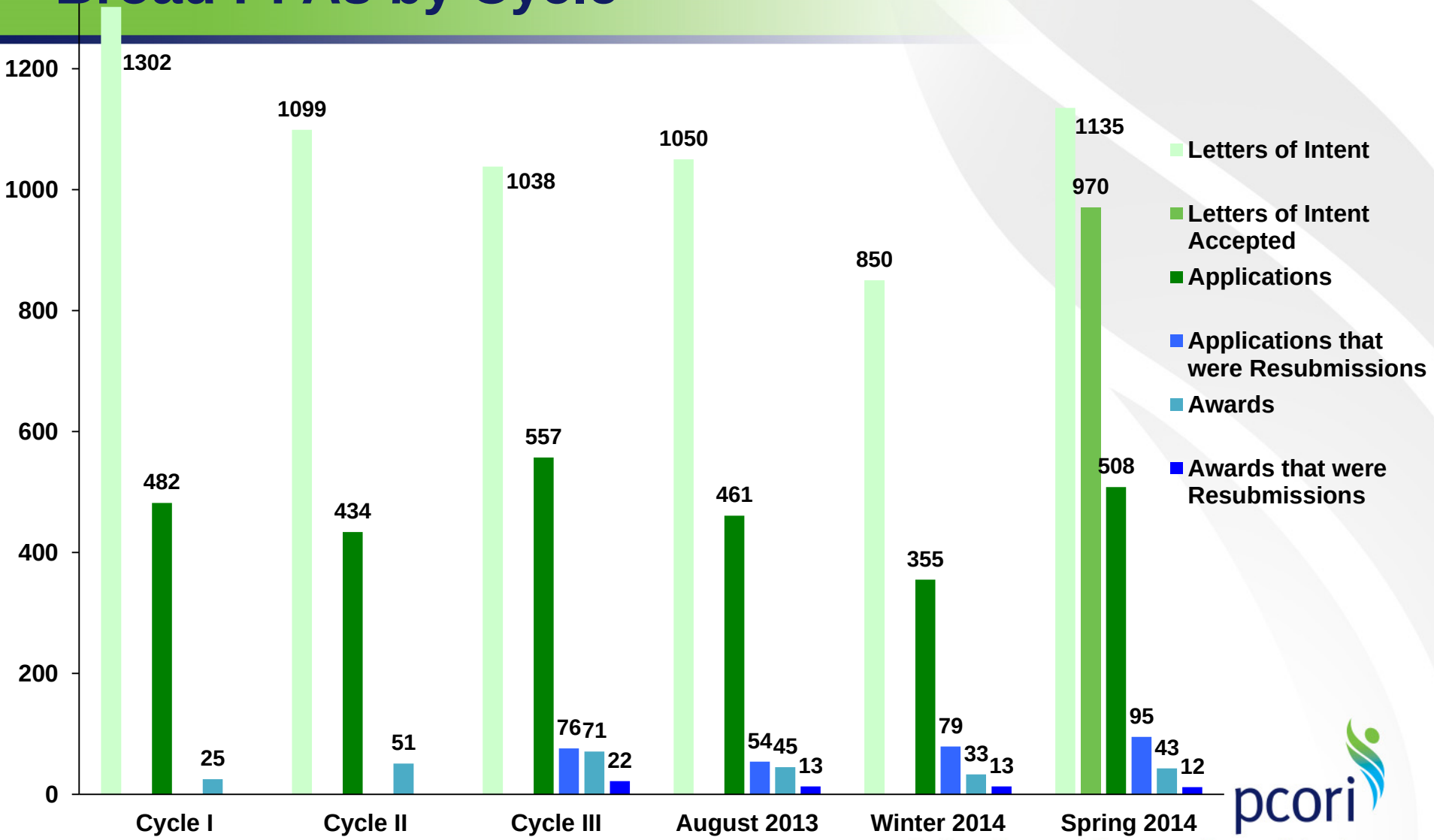
Responsiveness



Appendix

Additional Background Slides to Respond to Questions

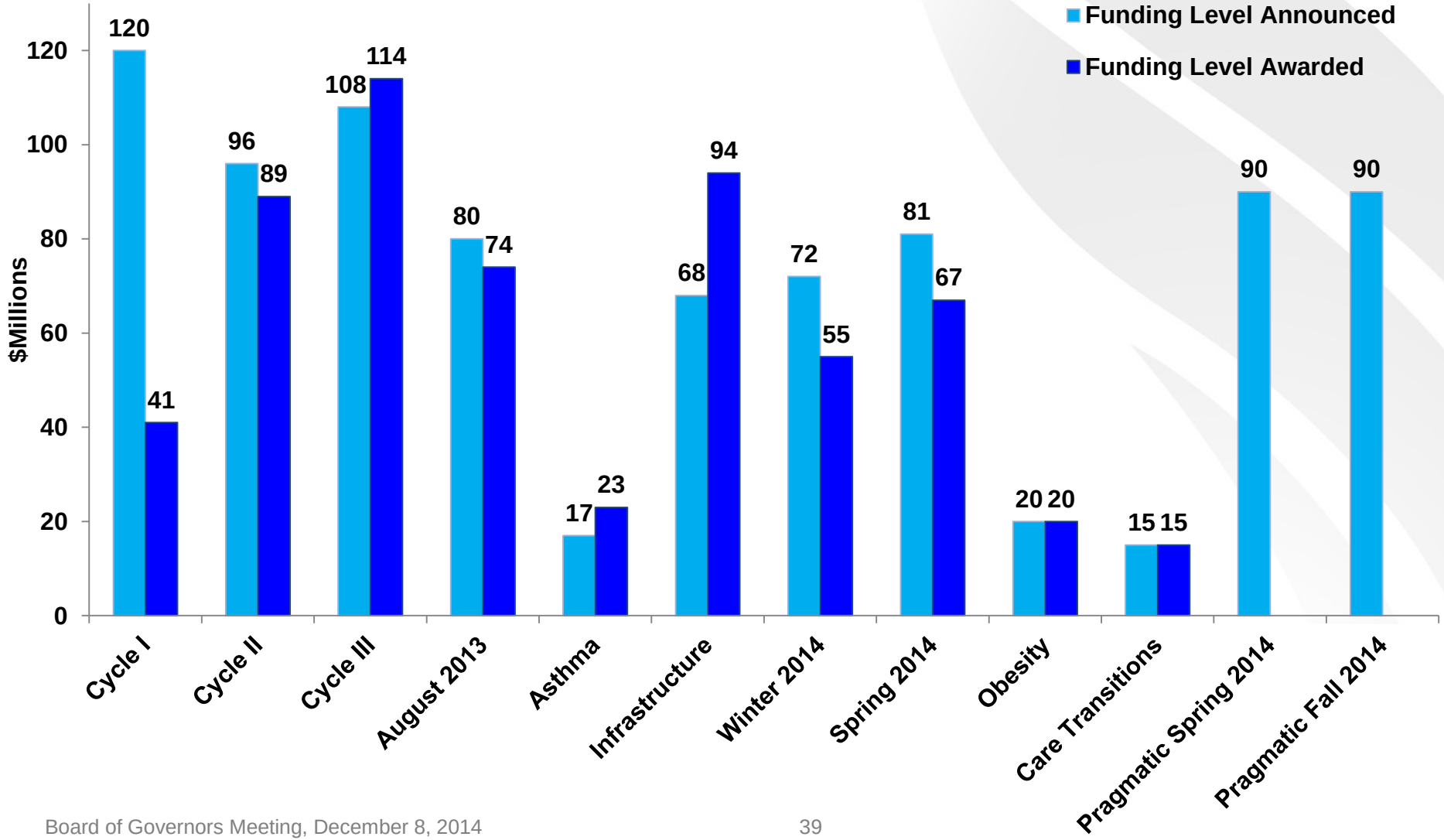
Number of LOIs, Applications, and Awards for Broad PFAs by Cycle



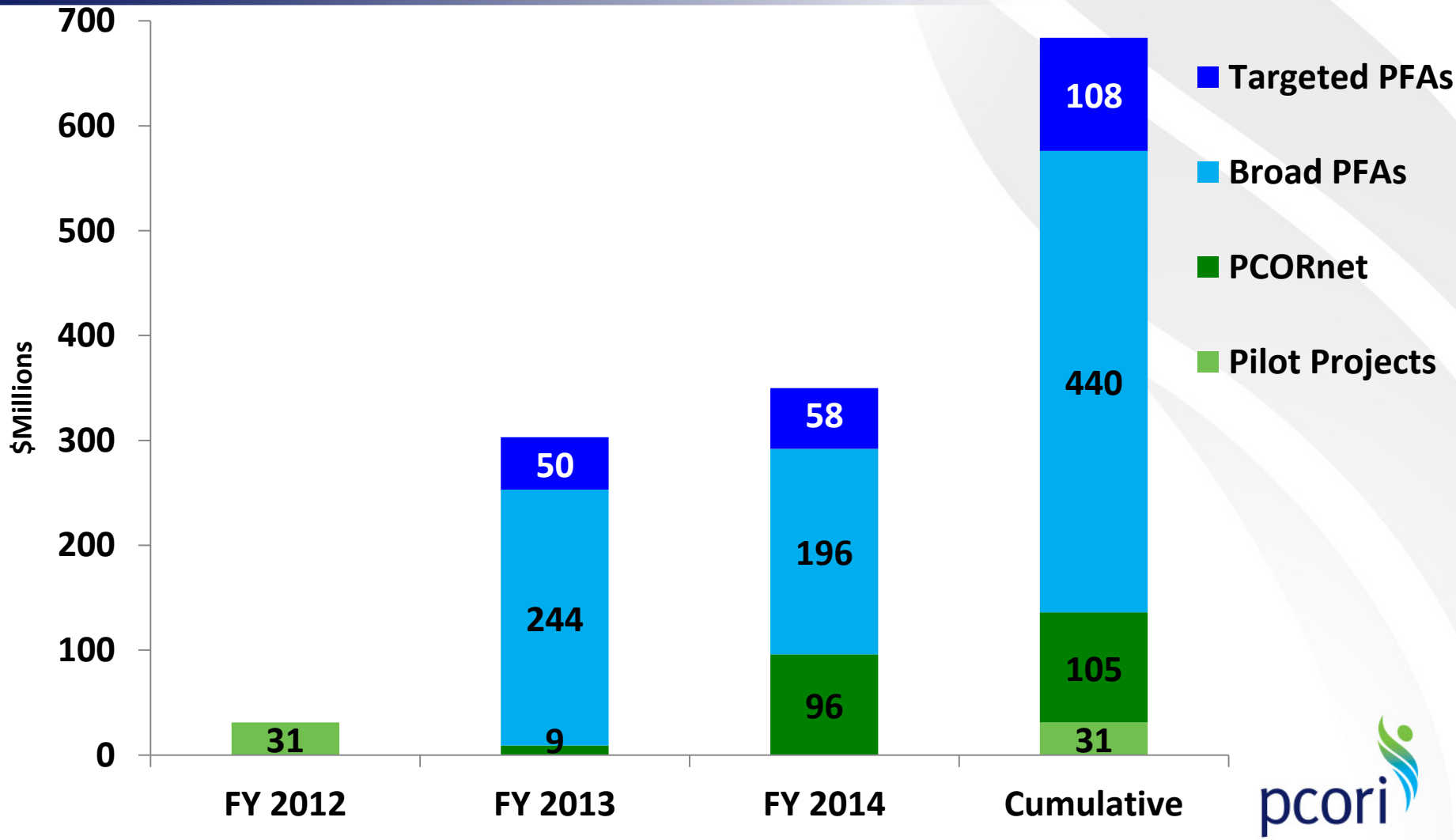
Status of Targeted Topics as of Q4 2014

Topic Status	FY 2014	FY 2015
Awarded	✓ Asthma (Q1): 8 Projects, $23M ✓ Falls (Q3): 1 Project, $30M ✓ Care Transitions (Q4): 1 Project, $15M ✓ Fibroids (Q4): 1 Project, $20M ✓ Obesity (Q4): 2 Projects, $20M	
Year-to-Date Total	5 Topics, 13 Projects, $108M	
To Be Awarded		• Hypertension: up to 2 Projects, $25M • 19 Pragmatic: up to 27 Projects, $270M
Posted	• 19 on Pragmatic Studies List	• 19 on Pragmatic Studies List
PFA in Development		
Nearing SOC and/or Board Vote	• Perinatal • Hepatitis C	
Under Consideration	95 Topics	95 Topics
Cumulative Total	5 Topics, 13 Projects, $108M	
Target for Year	5 Topics, ~14 Projects, $108M	Up to 20 Topics, 27 Projects, $337M

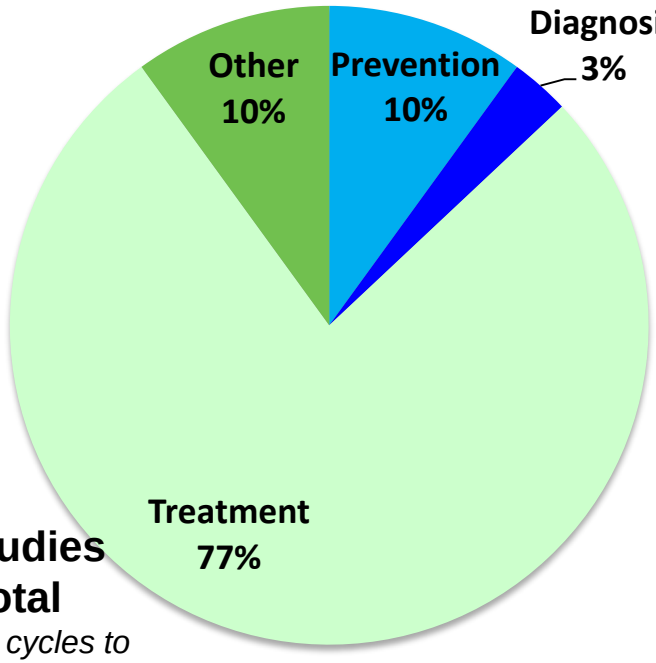
Funds Announced for Solicitations Compared with Funds Awarded



Funds Committed for External Research

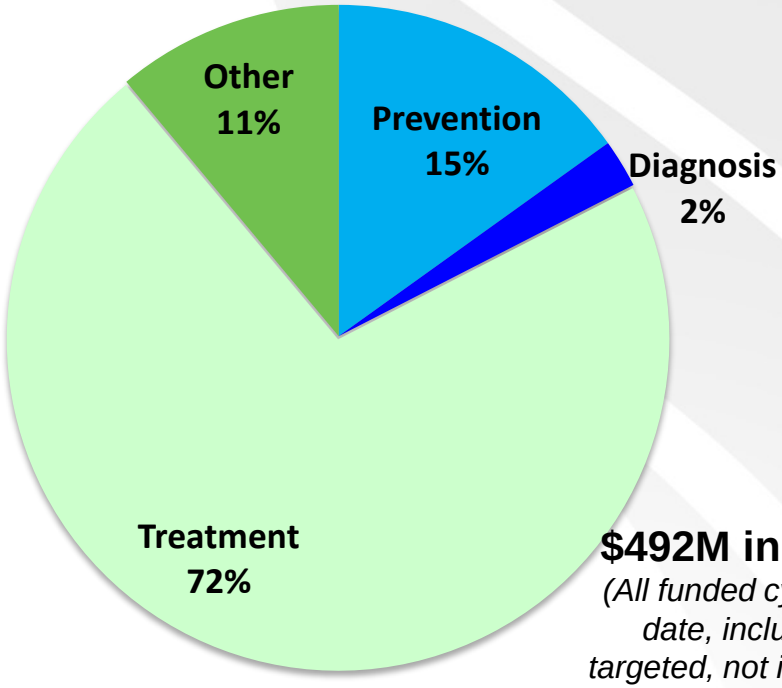


Composition of Current CER Portfolio (by number of projects and amount of funding)



223 Studies in Total

(All funded cycles to date, including targeted, not including Methods)



$492M in Total

(All funded cycles to date, including targeted, not including Methods)

Prevention Diagnosis Treatment Other

"Other" projects include screening, systems, and those overlapping categories

Progress of PCORnet at 6 months – Overall

Status of PCORnet Major Deliverables at Q4

✓ **Common data model**
- *Version 2 drafted and sent out for comment*
- *Proposed data domains: Prescriptions Dispensed, Patient-Reported Outcomes Common Measures, Lab Results, Conditions*

❑ **Governance policies – 13 in development (up from 8 last quarter)**
- *Two draft policies are at step 8 of 10 total steps (Introduction, Decision-making and Leadership)*
- *Four draft policies are at step 7 (Distributed Research Networks, Privacy and Confidentiality, Data Security, Confidential Business Information)*

✓ **Clinical trial**
- *Issued a limited PCORI Funding Announcement (PFA) soliciting a multi-network research plan for Aspirin Trial*
- *Principles of PCORnet Clinical Trials has been drafted and circulated to PCORI and PCORnet PIs for comment*
- *Clinical Trials Transformative Imitative (CTTI) Quality by Design Document is drafted and out for comment*

✓ **Steering Committee and Task Force – meetings occur regularly**

✓ **Communications**
- *IOM-PCORI Workshop Summary Published on Integrating Research and Practice: Health System Leaders Working Toward High-Value Care*
- *JAMIA Theme Issue published on PCORnet, including 11 articles on CDRNs and 1 PPRN summary article*
- *Health Affairs Big Data theme issue published, including 5 PCORnet-related articles*
- *PCORnet staff presentations at major conferences (Health Datapalooza, Association of Health Care Journalists)*

Progress of PCORnet: CDRNs at 6 months

Task	Number of CDRNs Completing Task
✓ ***PopMedNet capability*** established (DataMartClient Install Complete)	11/11
❑ Significant ***disease-specific collaborations*** across 2 or more CDRNs	2/11
❑ Submission of ETL/ADD form* for review by DSSNI operations team	5/11
❑ Performed initial, simple ***query on real data***	1/11
✓ Developed plans for ***streamlined Institutional Review Board***	11/11
✓ ***Patients currently engaged*** in leadership and/or advisory roles	11/11
✓ Plans for conducting ***clinical trials*** without disrupting healthcare operations	11/11
✓ Exploring approaches to ***complete data***	11/11

Meeting 100% of Milestones Due in Q4 – 2/11
Average Percent of Milestones Met – 85%

*ETL/ADD = Extract, Transform and Load / Annotated Data Dictionary

Progress of PCORnet: PPRNs at 6 months

9 COMMON CONDITION PPRNs

- ❑ **Data domains**:
  - ✓ demographic 9/9
  - ❑ vital signs 5/9
  - ❑ enrollment, diagnosis data, and encounter data 4/9
- ❑ **Patient portals**:
  - ✓ Developing 9/9
  - ❑ Beta launch 6/9
  - ❑ Launched 6/9
- ❑ **IRB Approval:** 8/9
- ✓ **Patient Engagement**: patients in governance 9/9

Meeting 100% of Milestones Due Q4: 2/9

8 RARE CONDITION PPRNs + CENA

- ❑ **Data domains**:
  - ❑ Demographic 5/9
  - ❑ vital signs 6/9
  - ❑ enrollment, diagnosis data, and encounter data: 8/9
- ❑ **Patient portals launched and enrolling patients:**
  - ❑ PPRNs 5/8
  - ❑ CENA organizations 5/9
- ❑ **IRB Approval:**
  - ❑ Full: 6/9
  - ❑ Partial: 3/9
- ✓ **Governance Structures Developed:** 9/9
- ✓ **Patient Engagement**: patients in governance 9/9

Meeting 100% of Milestones Due Q4 : 4/9

Average Percent of Milestones Met – All PPRNs – 92%

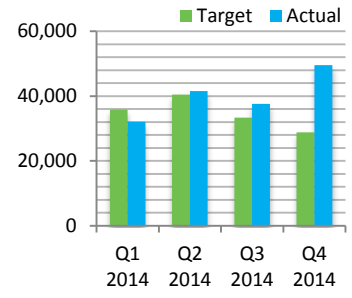
Communications: Website, E-mail, Social Media, and Media Coverage Metrics

Communications

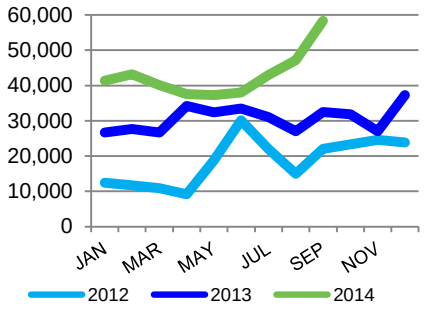
Website

Our Q4 unique website visitors numbers continue to beat our target.

Website Unique Traffic



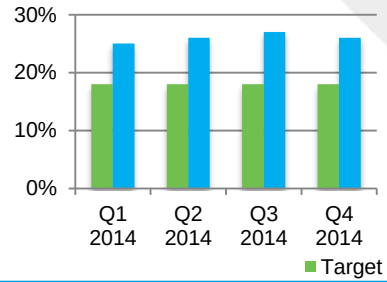
Monthly Unique Traffic



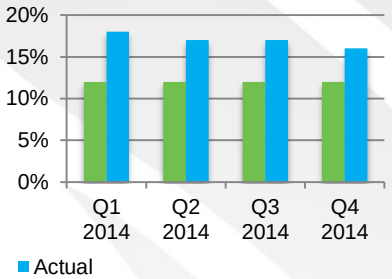
E-mail

We continue to exceed industry standards for open and click-through rates by a wide margin.

Email Open Rate



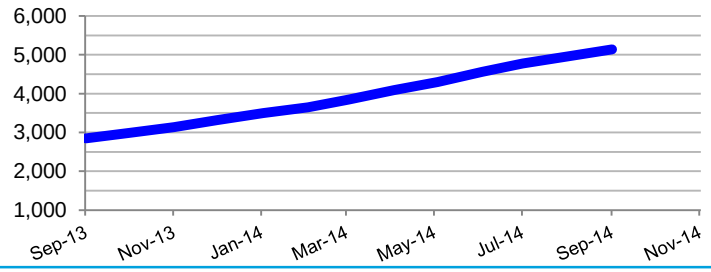
Email Click-Thru Rate



Social Media

Because our early Twitter stats were low, year-to-year comparisons against targets aren't useful. We're tracking follower growth and impressions and working on a more sophisticated reach analysis.

Monthly Twitter Follower Growth



Media Coverage

We continue to grow the number of mentions of our work in general/trade media.

	2012	2013	2014
Q1	-	45	150
Q2	47	48	212
Q3	46	89	149
Q4	26	117	166

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

Adherence by Standard Category (average across 3 cycles – 101 applications)

Standard Category	% Adherence
Research Questions	77%
Patient-Centeredness	96%
Data Integrity/ Rigorous Analyses	82%
Missing Data	100%
Heterogeneity of Treatment Effects	80%
Data Registries	67%
Data Networks	n/a
Causal Inference	95%
Adaptive/Bayesian Trials	n/a
Diagnostic Tests	80%
Systematic Reviews	0%

Adherence by Cycle (average across all standards)

August 2013 53 Applications	Winter 2014 23 Applications	Spring 2014 25 Applications
89%	92%	87%

**Does not include Methods Studies*

Committee Reports

- Governance Committee
- Science Overview Committee (SOC)
- Engagement, Dissemination and
- Implementation Committee (EDIC)
- Research Transformation Committee (RTC)
- Finance and Administration Committee (FAC)

Governance Committee Report

Steven Lipstein, MHA
Chair

Patient-Centered Outcomes Research Institute

Background: PCORI Board Evaluation

- In 2013, external consultants Marla Bobowick and Debbie Hechinger conducted an evaluation of the Board's policies, procedures and practices, and presented recommendations for enhancement.
- The Board endorsed almost all recommendations.
- Recommendations were implemented by the end of the first quarter of 2014.
- We continue to evaluate our progress and consider whether our governing policies, procedures and practices require further assessment and/or attention.

Key Findings and Actions Taken in 2013

Finding:

There is strong value in having clarity and consensus on PCORI's strategy, focus and priorities to support planning and development of metrics.

Actions taken:

1. Strategic Plan approved by the Board on November 18, 2013.

2. Dashboard to measure progress against the Strategic Plan implemented.

3. Annual review of the Strategic Plan.

Key Findings and Actions Taken in 2013

Findings:

The need for PCORI's early governance structures and processes to focus on operational issues should evolve to support a Board that focuses on governing.

Effective meeting preparation and practices can facilitate Board decision-making.

The 21 Board members respect each other, appreciate their diverse backgrounds, and work together collegially. The Board generally seeks consensus, which sometimes presents challenges.

Actions taken:

4. Board Meetings and Planning Sessions:
   - Number of in-person meetings of Board reduced.
   - Number of teleconference/webinar meetings of the Board increased.
   - Number of planning sessions reduced.

5. Board Meeting agendas:
   - Organized around goals of the Strategic Plan and structured to bring focus and discipline.
   - At each meeting, there is time for the Board to discuss issues, share new ideas and take up additional topics.

Key Findings and Actions Taken in 2013

Finding:

Strategies can be implemented to support the interaction between the Executive Director and the Board to enhance PCORI's governance and management.

Action taken:

6. The Chairperson, Vice-Chairperson, and Executive Director have standing weekly meetings to discuss Board/management issues and interactions, approaches to resolving problems, and to support effective communications.

Key Findings and Actions Taken in 2013

Findings:

Board members bring strong expertise that benefits PCORI. Staff members also have strong expertise but some are relatively new to PCORI.

Committees created during the start-up phase (when PCORI had no staff) should continue efforts to re-define their roles and relationships.

Actions taken:

7. Committees were re-constituted and re-chartered.

Board oversight committees include:

- Finance and Administration
- Governance
  - Audit and Conflict of Interest Sub-committee
  - Executive Evaluation and Compensation Sub-committee

Strategy Committees include:

- Science Oversight Committee
- Engagement, Dissemination and Implementation Committee
- Research Transformation Committee

PCORI Strategic Goals and Relationship to Strategy Committees

Goal	Generally aligns with	Chair and Vice Chair
Substantially increase the quantity, quality, and timeliness of useful, trustworthy information available to support health decisions.	Science Oversight Committee	Christine Goertz, DC, PhD, Chair
Speed the implementation and use of patient-centered outcomes research evidence.	Engagement, Dissemination, and Implementation Committee	Debra Barksdale, PhD, RN, Chair Robert Jesse, MD, PhD, Vice Chair
Influence clinical and health care research funded by others to be more patient-centered.	Research Transformation Committee	Freda Lewis-Hall, MD, Chair

Science Overview Committee Report

Christine Goertz, DC, PhD
Chair

Patient-Centered Outcomes Research Institute

SOC's Progress on the Objectives

Objective	2014 Deliverable/Action	Status
• **Research Portfolio Impact** – Establish a plan with key metrics to describe and assess the significance and potential impact of PCORI's research strategy, and the research portfolio.	Developed metrics and a plan to monitor and evaluate the research strategy, the overall research portfolio, merit review and patient involvement	✓
• **Planning and Process Standardization** – Develop and institute a plan, standard operating procedures (SOPs) and metrics for: (A) topic selection, (B) merit review, (C) selection committee process, and (D) monitoring and evaluating the research portfolio (1) fiduciary aspects and (2) identifying scientific gaps and opportunities.	Established plans and SOPs for all processes (A-D). Proposed evaluation metrics for each process. Once metrics are agreed upon, staff will propose PCORI benchmarks.	✓
• **Targeted Funding Announcements** – Develop priority topics for consideration including approval of two new targeted funding announcements for large priority-driven CER trials.	Approved 3 targeted announcements (obesity, transitional care and aspirin). Recommended 1 targeted announcement for FY2014 Board approval; 2 additional topics are near-term, including perinatal and a PCORnet observational study.	✓

Engagement, Dissemination, and Implementation Committee Report

Debra Barksdale, PhD, RN
Chair

Patient-Centered Outcomes Research Institute

EDIC's Progress on the Objectives

Objective	2014 Deliverable/Action	Status
• **Engagement Awards Program** - Enhance our primary research efforts by cultivating a PCOR-ready community, and encourage dissemination and implementation of patient engagement practices.	Establish close oversight to build-on a series of awards that are strategically related to help advance engagement and science objectives.	✓
• **Dissemination and Implementation (D&I)** - Facilitation of D&I through partnerships and infrastructure to engage patients and other stakeholder communities in D&I of PCOR. Coordinate with the Agency for Healthcare Research and Quality (AHRQ).	Complete the development of the PCORI Dissemination and Implementation Action Plan. Engage a wide and inclusive range of key patient and non-patient stakeholder groups such as nurses, payers, physicians, etc.	**In Progress**
• **Active Portfolio Management with the PCORI Engagement Rubri**c – Promote the rubric to advance engagement in research. Provide options for incorporating engagement into the research process for applicants, merit reviewers, awardees and Engagement Officers.	Integrate rubric into PFAs, Merit Review training, and applicant training. Hire, train, and implement the role of the Engagement Officers in monitoring research projects.	✓
• **Non-Patient Stakeholders** - Develop strategy for outreach to key non-patient stakeholder groups.	Provide input on the strategy for stakeholder outreach.	✓
• **D&I Evaluation -** Develop targets and milestones to speed implementation.	Provide guidance on the development of policies and practices to speed implementation.	**In Progress**

Research Transformation Committee Report

Freda Lewis-Hall, MD
Chair

Patient-Centered Outcomes Research Institute

RTC's Progress on 2014 Objectives

Objective	2014 Deliverable/Action	Status
Provide direction for the development of PCORnet	• Draft plan for Phase II • Draft strategy for sustainability	✓
Promote open science	• Develop PCORI strategy and implementation plan for open science	**In Progress**
Propose PCORI strategy for co-funding partnerships/alternative funding models, and evaluate the success of such models	• Develop strategy for PCORI collaborations • Assess collaborations under way	✓

Finance and Administration Committee Report

Kerry Barnett, JD
Chair

Patient-Centered Outcomes Research Institute

FAC's Progress on Objectives

Objective	FY2014 Deliverable (s)	Status
Monitor financial reports and budget variance to ensure timely implementation of programs and operations within budget and provide oversight to the creation of the proposed annual operating budget	FY2015 approved budget	**Completed.** Budget approved on time
Guide and monitor key issues related to PCORI's key position recruitment, attrition, job satisfaction and onboarding of new employees	Meet FY2014 staffing goal of 164; Improvements in employee effectiveness survey results	**Completed and ongoing.** Grew from 77 to 153 employees at end of FY2014 with balance of 164 pending start date as of 10/1/2014; saw significant improvement in annual survey results
Provide oversight to the development and revisions to key policies to ensure PCORI has effectiveness, efficiency and transparency in its operations	Key policies monitored and reviewed.	**Ongoing.** Continue to monitor policies to ensure they capture needs of organization. Revised pre- and post-award policies will be proceeding to the full Board this winter.
Guide and support the staff in developing its strategy specific to the financial issues related to PCORI's governing legislation	Key financial issues identified and plans developed	**Ongoing.** Plans being developed to review future funding.

PUBLIC COMMENT

Join the conversation on Twitter via #PCORI

pcori
Patient-Centered Outcomes Research Institute

BREAK

Join the conversation on Twitter via #PCORI

pcori
Patient-Centered Outcomes Research Institute

Science Oversight Committee Presentation: A Proposed 5-Year Research Funding Strategy

Christine Goertz, DC, PhD
Chair, Science Oversight Committee

Patient-Centered Outcomes Research Institute

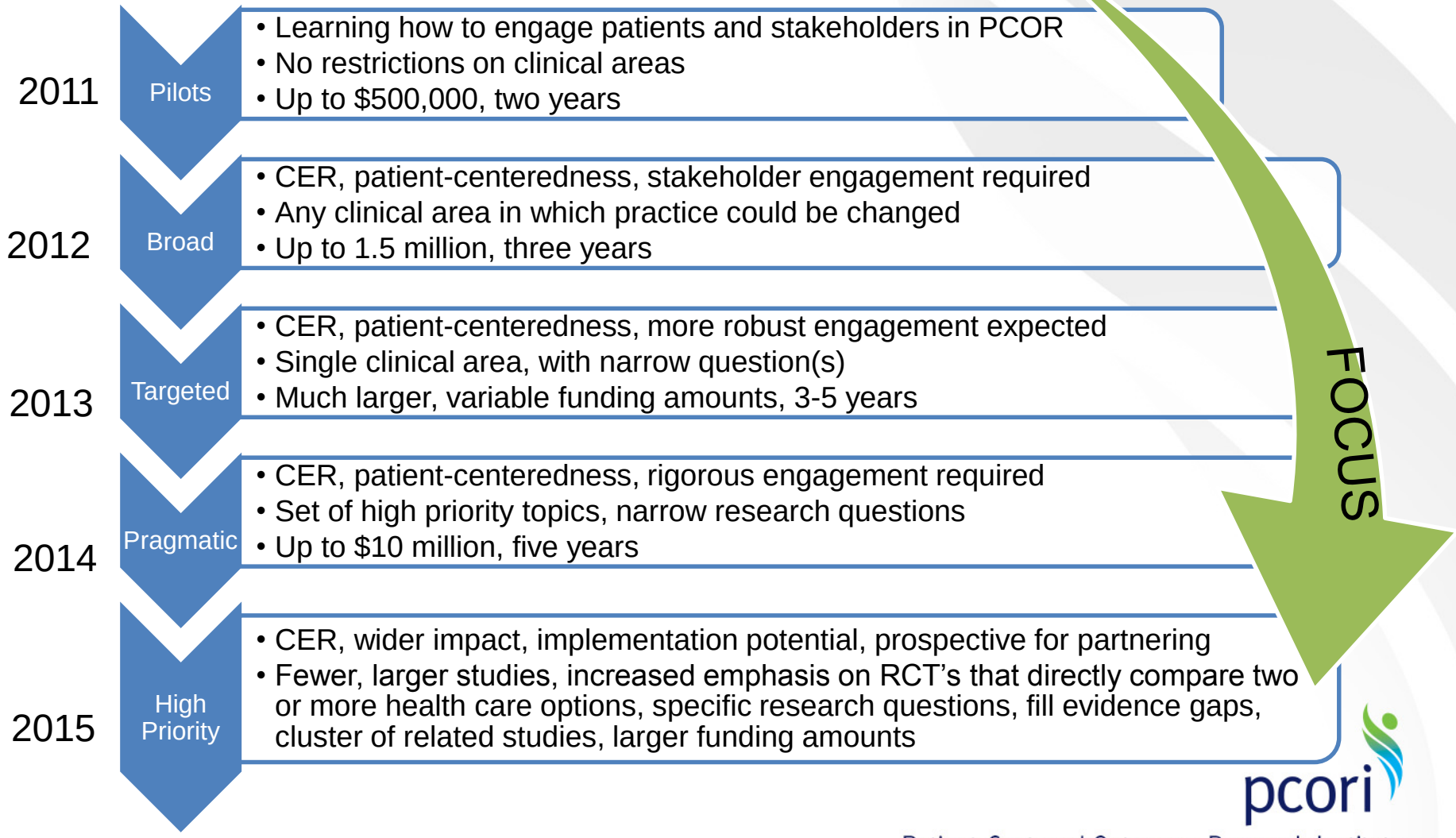
Discussion Question

- How well does this 5-year research funding strategy reflect the Board's vision?

Funding Vision:
Key Recommendations and Strategic Rationale

- Continue but taper broad research program
  - Continue to evaluate and build upon existing portfolio to identify and strategically manage clusters of research projects
- Continue Large Pragmatic Clinical Studies program
- Continue to target key topics, with increasing emphasis on comprehensive studies of selected **Priority Topics**
- Encourage the use of PCORnet, when appropriate

Laying the Groundwork for an Increasingly Focused Research Portfolio



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Continue to Fund Research Through Broad PFAs, but Taper Funding

- This line of funding solicits the research community's best ideas under PCORI's initial National Priorities for Research
- We have funded more than 250 studies with a maximum of $1.5M over up to 3 years
- The broad PFA remains the best way to tap into the creative cutting edge and to fund relatively young investigators
- ***Strategy going forward:*** because large clinical studies may be best suited to head-to-head comparisons in representative populations, funding will shift away from the broad PFA

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Total Dollar Amount Committed to Current Broad Research Portfolio

Broad PCORI Funding Announcements (PFAs)	Total Number of Research Awards	Total Funding (in $M)	% of Total Funding
Assessment of Options	83	$148	34%
Improving Healthcare Systems	53	$105	24%
Addressing Disparities	41	$71	16%
Methods	58	$54	13%
Communication and Dissemination	33	$57	13%
Broad PFA Total:	268	$437	100%

Note: This includes the Spring 2014 Cycle awards, which were announced on September 30, 2014.

Continue to Evaluate and Build Existing Research Portfolio

- PCORI is working to identify clusters of research in our existing portfolio as a way to actively manage our portfolio
- This is a key part of our detailed research strategy; the committee is working on developing the plan and timeline
- Provides opportunities to engage our stakeholders to help us identify gaps within a cluster

Continue PFAs for Large Pragmatic Studies

- Funding announcements stipulate:
  - Research topics that reflect national priorities for PCOR (PCORI, IOM, AHRQ)
  - Head-to-head comparisons in large, representative study populations
- We anticipate 5 funding cycles, up to $450M total commitment, and ~45 studies under way by late 2016
  - The first awards will be announced in February 2015
- **Strategic purpose:** Large Pragmatic Studies may be an effective way to pursue head-to-head comparisons of active interventions in representative populations

Large Pragmatic Studies

Cycle	# of LOIs Received	# of LOIs Accepted	# of Applications Received	Awards to be Announced
Spring 2014	231	35	35	February 2015
Fall 2014	168	24	16	Late April 2015
Winter 2015	132	TBD	TBD	July/August 2015

Targeted PFA Initiatives

- Funding announcements target a single research topic
- A Targeted PFA requires broad stakeholder support through the topic generation and prioritization process
- ***Strategic purpose:*** Targeted PFAs are important because they enable PCORI to move quickly to address topics of urgent national concern (e.g., Hepatitis C)
  - The number of projects in this program will likely increase but at a slower rate

Targeted Funding Announcement Topics Approved by the Board

Targeted Funding Announcement Topics	Funding Total	Status
Clinical Trial of a Multifactorial Fall Injury Prevention Strategy in Older Persons (Administered by NIA)	$30M	Awarded
Clinical Interventions to Reduce Hypertension Disparities (Administered through NHBLI)	$25M	Award in 2015
Treatment Options for African Americans and Hispanics/Latinos with Uncontrolled Asthma	$24M	Awarded
Obesity Treatment Options Set in Primary Care for Underserved Populations	$20M	Awarded
PCOR Treatment Options in Uterine Fibroids (Administered by AHRQ)	$20M	Awarded
Effectiveness of Transitional Care	$15M	Awarded
Optimal Maintenance Aspirin Dose for Patients with Coronary Artery Disease (PCORnet demonstration trial)	$10M	Award in FY 2015
Targeted PFA Total:	**$144M**	

Dedicate New Resources to a Few Targeted Priority Topics

- ***Strategic purpose:*** to make a lasting difference, focus a portion of our resources on key topics
- The **Priority Topic** Funding Announcements will:
  - Be limited to a small number of high priority topics (~10)
  - Make an extended commitment to each selected topic
  - Topics identified in variety of ways (e.g. key PCORI, ARRA funded CER studies and clusters of PCORI studies within the portfolio)
  - Fund clusters of studies around each Priority Topic
  - Enhance partnership opportunities (e.g. with federal agencies, patient, caregiver and clinician organizations; payers and the life sciences sector)

Proposed Percent Distribution Annual Funding Commitment

Commitment Period ($ in Millions, unless noted)				
Fiscal Period	**Research**	**Broad**	**Pragmatic**	**Targeted***
FY 2012-2013	$331			
FY 2014	$300	69%	0%	31%
FY 2015	$475	25%	55%	20%
FY 2016	$400			
FY 2017	$300			
FY 2018	$300			
FY 2019	$200	~30%	~20%	~50%
Cumulative Total (%)	**~$2.3 B (100%)**	**~$917 (40%)**	**~$655 (28%)**	**~$734 (32%)**

*This will be a blend of approaches to targeted funding; some may be single targeted topics and some may be Priority Topics (clusters of studies).

Encourage the Use of PCORnet, When Appropriate

- PCORnet to be “research ready” late 2015
- We can expect some applicants to the Broad, Large Pragmatic Studies, and Targeted PFAs will propose to use PCORnet
- Research in PCORnet will:
  - Be conducted in ‘real world’ settings
  - Take less time
  - Be less expensive

Funding Vision Recap

- Continue but taper Broad research program
  - Continue to evaluate and build upon existing portfolio to identify and strategically manage clusters of research projects
- Continue Large Pragmatic Clinical Studies program
- Continue to target key topics, with increasing emphasis on comprehensive studies of selected **Priority Topics**
- Encourage the use of PCORnet, when appropriate

Advantages of This Proposed 5 Year Funding Strategy

- It is evolutionary and consistent with strategy to date, building on existing investments
- Continues commitment to research (albeit at reduced level)
- Leverages our multi-stakeholder Advisory Panels prioritization efforts
- Engages our stakeholder community and builds off momentum created by its programs
- Maintains flexibility to respond to opportunity
- Enhances our opportunities for partnering with federal agencies and private institutions
- Provides for concentration of future resources to maximize adoption into practice and improvement of patient outcomes

Caveats of this Proposed 5 Year Funding Strategy

- While the Broad PFAs will continue, funding will be at a lower level
- The Large Pragmatic Studies PFA strategy depends on a continuing supply of good questions and experienced researchers
- Both the PFAs for Priority Topics and PCORnet research funding strategies are untried

Final Comment

This proposed funding strategy is the continuing evolution of our shift to larger, targeted, more stakeholder-based patient-centered outcomes research.

Discussion Question

- How well does this 5-year research funding strategy reflect the Board's vision?

Proposal for a Hepatitis C Targeted PFA

Bryan Luce, PhD, MBA
Chief Science Officer, PCORI

Patient-Centered Outcomes Research Institute

Clinical Impact of Hepatitis C

- An estimated 2.7-3.9 million cases in US, majority undiagnosed
- Peak incidence 29,000 in 2002; incidence now ~18,000 per year over the past decade
- About 1/3 will develop chronic liver disease if untreated
  - ~ 20 years to develop liver cirrhosis
  - If SVR achieved on interferon regimens, 2% develop cirrhosis
- Accounts for about ¼ of all US cases of cirrhosis and liver cancer
- No evidence about the effect of new direct-acting antivirals (DAAs) on long-term outcomes – relapse, progression of liver disease or in 'real world' settings
  - Reinfection rates not known

SVR = Sustained viral response

Current and Pending Treatment Options

- 2011: FDA approves telaprevir (Incivek) and boceprevir (Victrelis) for use in “triple therapy” with interferon
  - SVR rates increased from 50% to 70%
- 2013: FDA approves simeprevir (Olysio) and sofosbuvir (Sovaldi)
  - Both are used in triple therapy for genotype 1
  - Sofosbuvir/ribavirin double therapy for genotypes 2 and 3
- October 2014: FDA approves combination drug sofosbuvir/ledipasvir (Harvoni) for genotype 1
- Early 2015: Several additional agents anticipated for approval

Why a Targeted PFA on this Topic?

- Hepatitis C constitutes a substantial burden of illness
  - Prevalence, suffering, and mortality (including advanced liver disease); also patient and societal treatment costs

- Because of new, potentially transformational medications and as yet no long-term, 'real world' effectiveness or *comparative* effectiveness data, PCORI has an opportunity to inform practice through research

- A large and diverse number of stakeholders identified important clinical questions that address practical clinical issues and outcomes important to patients

Stakeholder Input

- **PCORI's Assessment of Options Advisory Panel** met September 19th
  - Panelists ranked hepatitis C #1

- **Multi-Stakeholder workshop** held October 17th
  - **Recommendation:** Four research topic areas received strong support in a multi-round voting process

Stakeholder Meeting Attendees

Stakeholder Group	Hepatitis C Workshop	Advisory Panel on APDTO
Clinicians	7	4
Coalition	1	0
Federal	5	0
Industry	6	3
Patients	8	7
Payers	4	1
Purchasers	1	1
Researchers	1	5
Systems	2	0
Total	**35**	**21**

Organizations Represented at the Hepatitis C Workshop

- AbbVie
- Academy of Managed Care Pharmacy
- Agency for Healthcare Research and Quality
- American Academy of Family Physicians
- American Association for the Study of Liver Diseases
- American Association of Nurse Practitioners
- American College of Physicians
- American Gastroenterological Association
- American Public Health Association
- America's Health Insurance Plans
- Blue Cross and Blue Shield Association
- Bristol-Myers Squibb
- Caring Ambassadors Program
- Centers for Disease Control and Prevention
- Centers for Medicare and Medicaid Services
- Food and Drug Administration
- Gilead Sciences

Organizations Represented at the Hepatitis C Workshop (continued)

- Global Liver Institute
- Hepatitis C Association
- Hepatitis C Mentor and Support Group
- Hepatitis Education Project
- Hepatitis Foundation International
- Infectious Diseases Society of America
- Janssen
- Medicaid Medical Directors Network
- Merck & Co.
- National Business Group on Health
- National Institutes of Health
- National Pharmaceutical Council
- National Viral Hepatitis Roundtable
- OHSU/Drug Effectiveness Review Program
- Partnership to Improve Patient Care
- UnitedHealth Group
- Veterans Health Administration
- Veterans Health Council

Four Key Questions Emerged

1. Screening and Diagnosis

- Which screening methods and testing strategies in which settings lead to the best detection rates?

2. Care Delivery

- What is the comparative effectiveness of intermediate care management versus more intensive, integrated care management strategies on treatment medication adherence and sustained viral response in patients with chronic hepatitis C infection who are at high risk of non-adherence?

Four Key Questions Emerged

3. Head-to-Head Treatment Comparisons of New Therapies

- What are the trade-offs between long-term virologic response and adverse effects for different new regimens of oral antiviral medications, including sofosbuvir/ledipasvir and other emerging therapies for treatment of hepatitis C infection in various patient populations?

Note: Because of the rapidly changing treatment landscape, PCORI will fund only *adaptive* head-to-head designed trials in order to integrate newly approved drugs to treat HCV.

Four Key Questions Emerged

4. Studies of the Effect of Delayed Treatment in Newly Diagnosed Hepatitis C without Serious Liver Disease

- What are the comparative benefits and harms of starting treatment immediately after a diagnosis of HCV infection (in the absence of serious liver disease) versus active surveillance (starting treatment only if the patient shows progression of liver disease or other manifestations of hepatitis C infection)?
  - The study population would be all newly diagnosed patients found to be at relatively low risk of progressive liver disease.

Note: Rather than proposing a randomized comparison, PCORI will encourage rigorous, prospective observational outcomes studies based on practice and coverage variation (e.g., insurance coverage policies that differ from state to state).

Recommendation for Targeted PFA

- Up to four large clinical studies to test the comparative effectiveness of alternative approaches to:
  - screening in various patient populations
  - care delivery choices for Hepatitis C treatment
  - timing of treatment (observational)
  - active treatment choices

- Recommended by staff, endorsed by the SOC for recommendation to the Board of Governors

Budget and Project Period

- PCORI proposes to commit up to $50 million in total costs to fund up to four studies
- Maximum for any single study: $20 million total costs
- It is expected that project budgets and duration will vary substantially, depending on the study approach, needs for recruitment and/or primary data collection, required length of follow-up, and analytic complexity

Patient Centered and Clinical Outcomes (not exhaustive)

- Screening performance score
- Sustained viral response (SVR), time to SVR, adherence, tolerability, long term outcomes (cirrhosis, decompensated cirrhosis, utilization)
- Patient-reported outcomes (e.g., quality of life, cognitive function, depression, satisfaction, fatigue)
- Utilization of health care
- Evidence of liver fibrosis or cirrhosis
- Long term relapse
- Risk of reinfection

Target Populations

Adult populations that may include:

- Persons defined solely by their birth cohort and without signs of active liver disease – (approximately 75% of all infected persons born during 1945- 1965)
- Hard-to-treat patients as defined by CDC (e.g., genotype 1a with cirrhosis patients, those with significant co-morbidities, those likely to be re-infected)
- Hard-to-reach populations (e.g., homeless, people with risky behaviors) and other target populations including incarcerated adults and pregnant women

Proposed Timeline

Action	Date
Release Date	February 5, 2015
PCORI Online System Opens	February 5, 2015
Applicant Town Hall Session (Webinar)	February 15, 2015
Letter of Intent Due	March 6, 2015 by 5:00 PM (EST)
Application Deadline	May 5, 2015 by 5:00 PM (EST)
Merit Review	Week of August 4, 2015
Awards Announced	September 2015

Board Vote: Hepatitis C PFA

Call for a Motion to:	• **Approve $50M for Hepatitis C PFA**
Call for the Motion to Be Seconded:	• **Second the Motion** • **If further discussion, may propose an Amendment to the Motion or an Alternative Motion**
Roll Call:	• **Vote to Approve the Final Motion** • **Ask for votes in favor, opposed, and abstentions**

Development of a PCORI Funding Announcement for PCORnet Phase II

Joe Selby, MD, MPH
Executive Director
Rachael Fleurence, PhD
PCORI Program Director CER Methods and Infrastructure

Patient-Centered Outcomes Research Institute

PCORnet Phase II Goals

- Highly engaged patients, researchers, clinicians, health systems participating in network governance, topic generation
- Analysis-ready standardized data, use of the PCORnet Common Data Model and preserving strong privacy and data security protections
- An infrastructure for supporting clinical trials embedded within network delivery systems
- An efficient research oversight framework that fosters public trust in research
- A collaborative community that attracts a diverse set of researchers, funders and other networks
- Research networks that are sustainable by end of Phase II

PCORnet Phase II – Planned Projected Capacity

The planned projected capacity for PCORnet by the end of Phase II is as follows:

Phase II Year	Rapid Queries (#, cumulative)	Clinical Trials (#, cumulative)	Observational Studies (#, cumulative)
Year 1	50	5	10
Year 2	100	10	20
Year 3	200	15	40

The number of studies are for projection purposes only, and describe the projected capacity of the PCORnet infrastructure by the end of Year 3.

Highlights of Phase II Announcements

- Emphasis on PCORnet as a national resource to support multi-site observational and interventional research more efficiently and at lower cost
- Governance in place at CDRNs and PPRNs to oversee rapid queries, observational studies and clinical trials
- Collaborations with CMS, Sentinel, health plans to obtain comprehensive patient data
- Efficient use of Natural Language Processing (NLP) to leverage more information from EHRs
- Explicit plans for collaboration with CTSAs where they co-exist
- Significantly increased collaborations between PPRNs and CDRNs
- Sustainability plans for the networks beyond Phase II

Numbers of Awards and Eligibility

- Up to 13 awards for CDRNs
- Up to 22 awards for PPRNs
- All current awardees are eligible to apply
- New applicants may apply, provided they can meet baseline requirements within 6 months of start of Phase II

Funding for CDRNs and PPRNs Over 3-Year Phase II (2015-2018)*

	3-year Total Costs (per network)	3-year Total Costs (assumes 13 CDRNs and 22 PPRNs)
CDRNs	$8,750,000	$113,750,000
PPRNs	$1,680,000	$36,960,000
Total Phase II (CDRNs + PPRNs)		$150,710,000

*This proposed level of funding was presented to the Board of Governors on September 15th, 2014, and endorsed by the RTC in a vote on December 5th 2014.

Application Review Criteria for Phase II

- Successful achievement of Phase I requirements
  - New applicants must provide evidence of capability of meeting Phase I requirements within 6-months of their contract award
- Strength of proposed activities to meet Phase II goals
  - Stakeholder Engagement, Analysis-Ready Data, Clinical Trial Infrastructure, Research Oversight Framework, Collaboration and Sustainability
- Evidence of a solid staffing and management plan
  - Requiring two co-PIs per network at total of 65% time
- Evidence of an efficient budget

Application Review Process

- Competitive Letters of Intent (LOIs)
  - Submitted by all applicants and reviewed by program staff
- Merit Review
  - Full applications will be reviewed by review panels that include program staff and invited external expert reviewers, including patients
  - Reviews will use review criteria described in prior slide
- Post-Panel Review
  - Applications and scores reviewed by program staff
  - Applications recommended to a Selection Committee
  - Recommended slate proposed for approval to the Board of Governors

Proposed PFA Timeline

Date	Action
November 2014	PFA concepts presented to RTC
December 2014	Issue CDRN and PPRN PFAs
January 2015	Competitive LOI received
January 2015	Applicants invited to submit
April 2015	Applications due to PCORI
June 2015	Merit review
July 2015	Selection Committee and Approval from Board of Governors
August 2015	Awards announced

Board Vote: Release of PCORI Funding Announcement for PCORnet Phase II

Call for a Motion to:	• **Approve** the release of PCORI Funding Announcement for PCORnet Phase II (CDRNs and PPRNs)
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

Methodology Committee Update

Robin Newhouse, PhD, RN
Chair

Patient-Centered Outcomes Research Institute

Session Topics and Objectives

What are we going to cover today?

Methodology Standards	• Dissemination and Implementation Update • Review of existing standards
New Standards in Development	• Progress update on development of new standards for research designs using clusters and complex interventions
Methods Monitoring in the Portfolio	• Discuss current activities and next steps
Network Research Methods Work Group	• Highlight Network Methods Work Group activities on Distributed Analyses
Other Updates	• Value of Information presentation volunteered • Decision Sciences Expert Meeting

Welcome to New MC Members

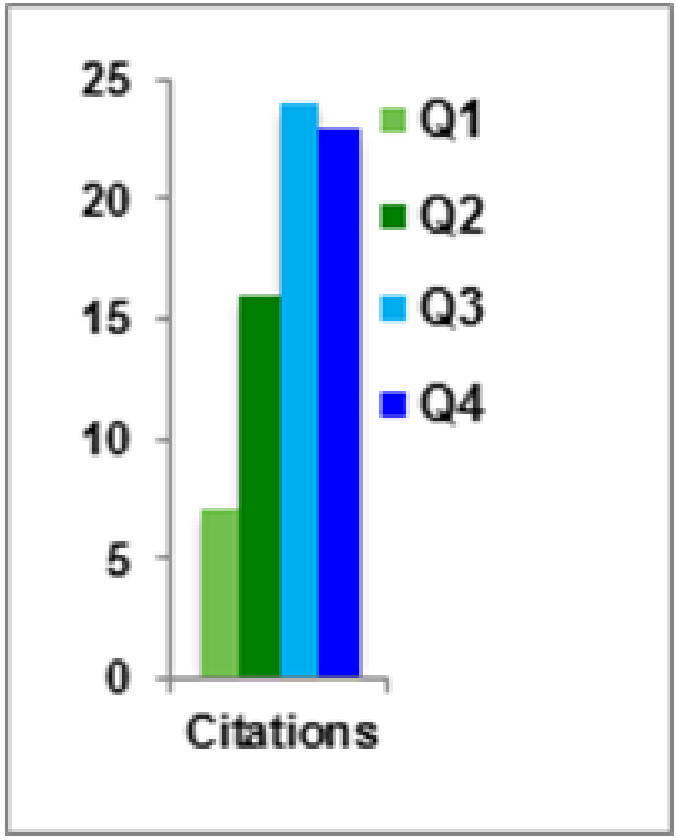
- Cynthia Girman, DrPH
- Sally Morton, PhD
- Neil Powe, MD

Dissemination and Implementation of the Methodology Standards: *Activities underway since September 2014*

- **PCORI Standards Work Group:**
  - Continue to guide operationalization of Standards within PCORI
  - Promote training of staff and merit reviewers
- **Dissemination Activities:**
  - Continue outreach to cultivate partnerships
  - Staff are tracking journal and scholarly work published on standards and the report

Dissemination and Implementation of the Methodology Standards: Uptake Indicators

- PCORI Methods Citations – report from Medical Librarian, graphic from the Dashboard is below



Dissemination and Implementation of the Methodology Standards: *Activities underway since September 2014*

- **Standards Training Activities:**
  - CME/CE training task order to be released in early December; Deliverable expected May 2015
  - RFP for course materials aligned with the standards to be released by mid December 2014; Deliverable expected by early Summer 2015
  - Discussions with AHRQ are underway on how to collaborate with their R25 awardees for testing and implementing curricula
  - Developing training material for release in early 2015

Review of Existing Methodology Standards

- All 47 existing standards will be reviewed by PCORI staff (one team for each Standards category) with the purpose of clarifying standards to facilitate adherence
  - Brief operational examples will be developed and, in some cases, wording changes recommended
  - Two MC members (including original member(s) for category) will review staff recommendations
  - Full MC will discuss and approve
- Projected timeframe: January – September 2015

Development of New Methodology Standards

- Standards for Designs with Clusters Meeting
  - **Goals:** To develop and help to refine a set of standards
  - Planning a Cluster Research Design meeting in February 2015
- Standards for Complex Interventions
  - **Goals:** To develop and create a definition of complex interventions
  - Definitional work to be performed and plan for future standard development work in Q2 2015
  - MC lead: Brian Mittman

Methods Monitoring in the Portfolio

- **Ongoing Monitoring of PCORI Methods Funding Program**
  - At the November Methodology Committee meeting, the Methods portfolio was reviewed and gaps and future directions were discussed
- **Clinical Trials Advisory Panel Subcommittees**
  - Newly appointed and formed for consultation on clinical trials
    - Subcommittee on Recruitment, Accrual, and Retention (RAR) – David Meltzer
    - Subcommittee on Monitoring of Funded Clinical Trials (MFCT) – [Still being formed; MC member TBD]
    - Subcommittee on Standardization of Complex Concepts and their Terminology (SCCT) – Mary Tinetti and Robin Newhouse

Methods Consultation Panels

- Methodological challenges present in virtually all clinical trials and observational studies PCORI reviews and funds
- MC proposed additional methodological review to ensure/enhance rigor of study designs
  - Recruited pool of external methods experts who will review applications for methodological issues only

Methods Consultation Panels

- Piloted as part of evaluation of Spring 2014 Large Pragmatic Studies applications
  - The 20 most meritorious Pragmatic applications emerging from Merit Review will be evaluated through this process
  - 3 panels of 6 experts convening in mid-December
  - Will provide input to PCORI staff about potential modifications and improvements to proposed study designs; inform staff decision-making about slate recommendations to the Board of Governor's Selection Committee for funded projects
- PCORI will learn from this process to make determinations of its use in future funding cycles
  - MC leads: Steve Goodman, Sally Morton

Update: Network Research Methods Work Group

- Promising Models and Future Research Needs Related to the Conduct of Distributed Analyses in Data Networks
  - **Progress:** Fall 2014 invitational workshop for representatives of data networks that currently conduct distributed analyses
    - Identified networks currently engaged in doing distributed data analyses and relevant individuals within those networks to invite to the workshop
    - **Workshop held December 4, 2014**
    - Created a template from the structured abstracts for meeting invitees and starting a white paper
  - MC lead**:** Sebastian Schneeweiss

Other Updates

- Decision Sciences Expert Meeting
  - Plan to convene a group of experts in April 2015
  - Contract to conduct this meeting has been awarded
  - MC leads: David Flum, Mark Helfand, David Meltzer
- Open Science
  - MC lead: Steve Goodman
- Recommend : Value of Information (VOI)
  - MC members will discuss opportunities in VOI with the BOG
  - MC lead: David Meltzer
- GAO nominations for the final position on the Methodology Committee (health informatics expert) closed on November 17th

Methodology Committee

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

BREAK

Join the conversation on Twitter via #PCORI

Engagement Update

Jean Slutsky, PA, MSPH
Chief Engagement and Dissemination Officer

Patient-Centered Outcomes Research Institute

Outline

- Overview
- Stakeholder Engagement
- Engagement Awards
- Pipeline to Proposals
- Ambassadors Program
- Training

Overview

- Assumed the Chief Engagement and Dissemination Officer position on March 17, 2014
  - Performance plan element to increase interconnectivity of Engagement with science
  - Evaluate where we are on all Engagement investments
- All programs had begun, some more mature and others just starting
- Engagement programs are a significant part of PCORI's Dashboard
- Over the last five months we have held three strategic planning sessions for the Engagement Awards program, Pipeline to Proposals Program, and the Ambassadors Program
- Engagement staff meetings with Science Staff
- Overall Engagement Strategic Planning session planned for later this month

Overview (continued)

- Significant patient and caregiver engagement activities have moved from planning to full scale implementation
- Since April 2014, rates of involvement have increased among clinicians, hospital/health systems, health plans, training institutions, and policymakers
- Significant efforts have been placed towards:
  - Collaborating with the Science team on multi-stakeholder events (workshops and roundtables) which inform the development of funding announcements
  - Soliciting prioritized research topics from key stakeholder communities
  - Setting up informational meetings between PCORI leadership and representatives of key stakeholder groups
  - Outreach to increase involvement among all stakeholder communities in PCORI's merit review process

Overview of Stakeholder Engagement

PCORI Stakeholders

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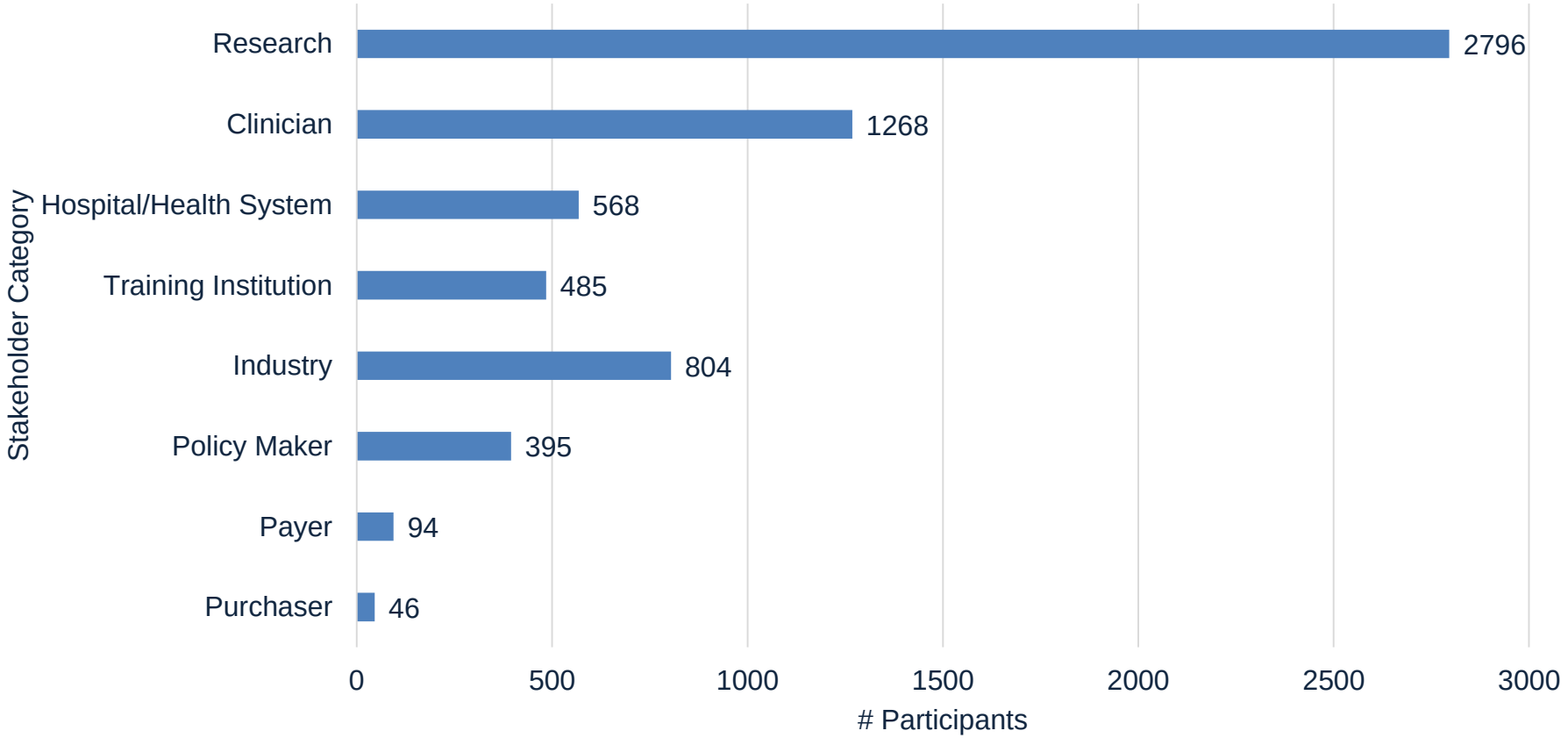
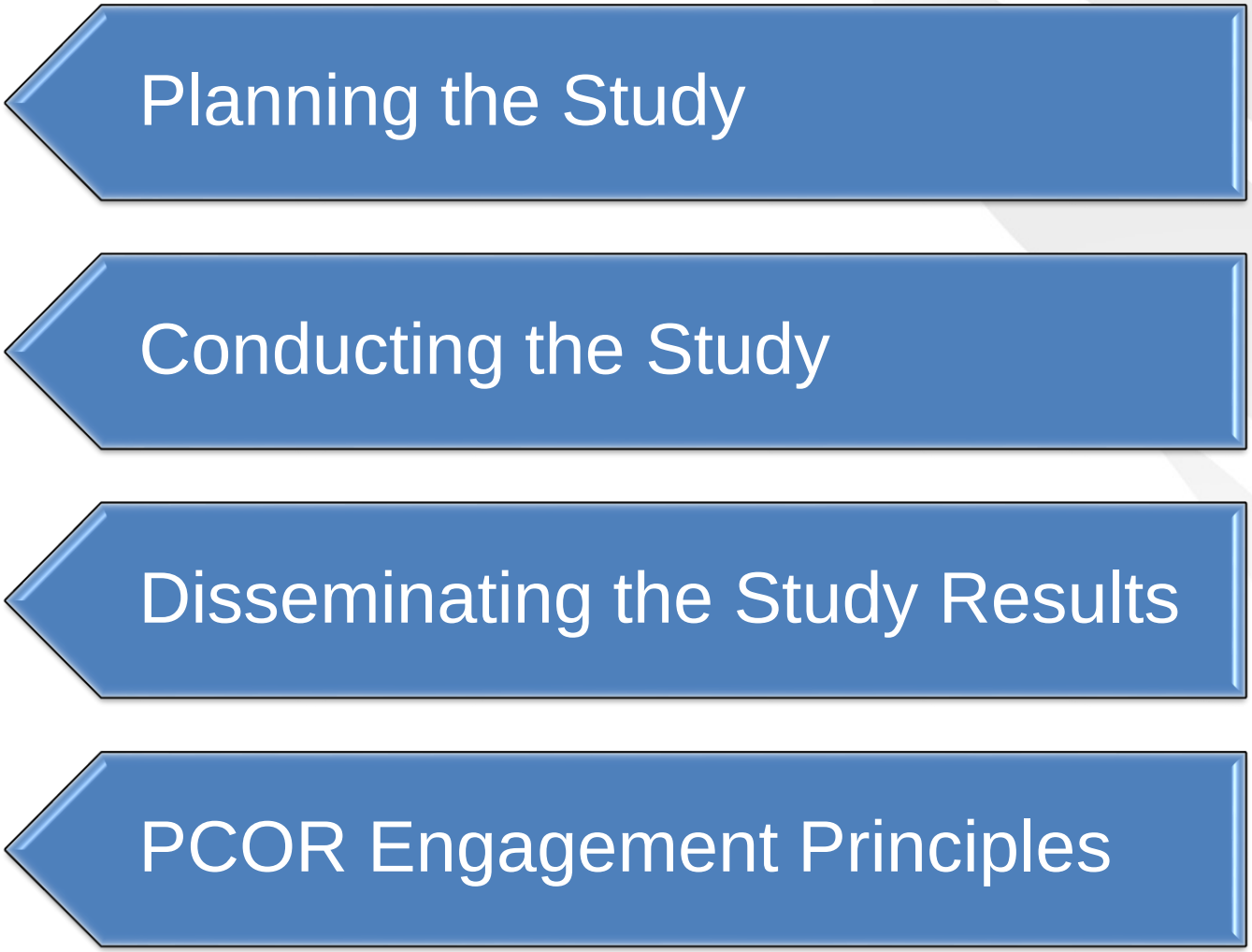
Total Stakeholder Participation

January 1, 2013- September 31, 2014 (N= 6,456)



Data represents stakeholder participation in Advisory Panels, Award Notifications, 1:1 Meetings, Merit Review, Speakers Bureau, Webinars, and Regional Workshops

Supporting Patient and Family Engagement in Patient-Centered Outcomes Research (PCOR); Implementation of the Rubric



The PCORI Engagement Rubric: Planning the Study

Planning the St

Formulating Research Questions and Study

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Formulating Research Questions and Study Design

Patient partners participate in:

- **Identifying the topic and developing the research question to be studied.**
- **Creating the intervention to be studied (if applicable) and identifying comparators.**
- **Identifying the goals or outcomes of the interventions to be studied.**
- **Defining essential characteristics of study participants.**
- **Other study design and preparation.**

The PCORI Engagement Rubric: PCOR Engagement Principles

PCOR Engageme

Reciprocal Relationsh

Reciprocal Relationships

The roles and decision-making authority of all research partners, including patient partners, are clearly stated.

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Engagement Officers: Ensuring Meaningful Engagement in Research by Partnering with Science

- **Where we have been:**
  - Development phase of the concept and operationalization of Engagement Officers
  - Engagement Officer pilot initiative with one Science program area for past 12 months
- **Where we are now:**
  - Three full time Engagement Officers deployed in 3 science program areas focusing primarily on engagement in targeted awards, pragmatic trials and PCORnet awards
- **Where we are going:**
  - Assess the value of the Engagement Officer and determine optimal number of Engagement Officers to assure adequate support and monitoring of engagement in PCORI funded projects

Engagement Officers Collaboration with Science Project Officers

Merit Review

LOI review

Observation of review

Slate selection

Pre-Award

Review and modify milestones

Ensure adherence to Engagement Methodology Standards

Augment engagement

Post-Award

Engagement milestones

Kick-off calls

Interim report review

Interim calls

Site visits

Patient and stakeholder interviews

Eugene Washington PCORI Engagement Awards

Eugene Washington PCORI Engagement Awards

- **Program Growth**
  - Lia Hotchkiss, Director
  - Shivonne Laird, Program Officer
  - Arielle Altman, National Urban Fellow
- **Strengthened Program Infrastructure**
  - More robust review process
  - Moved from rolling to quarterly review cycle
  - Added conference support
  - Working closely with PCORI Contracts Management and Administration to review applications and make awards in a timely way
- **Increased Awareness About Program & Provided Additional Guidance to Applicant Community**
  - Updates to PCORI website
  - Public webinar
  - Fact sheet
  - Meeting and calls

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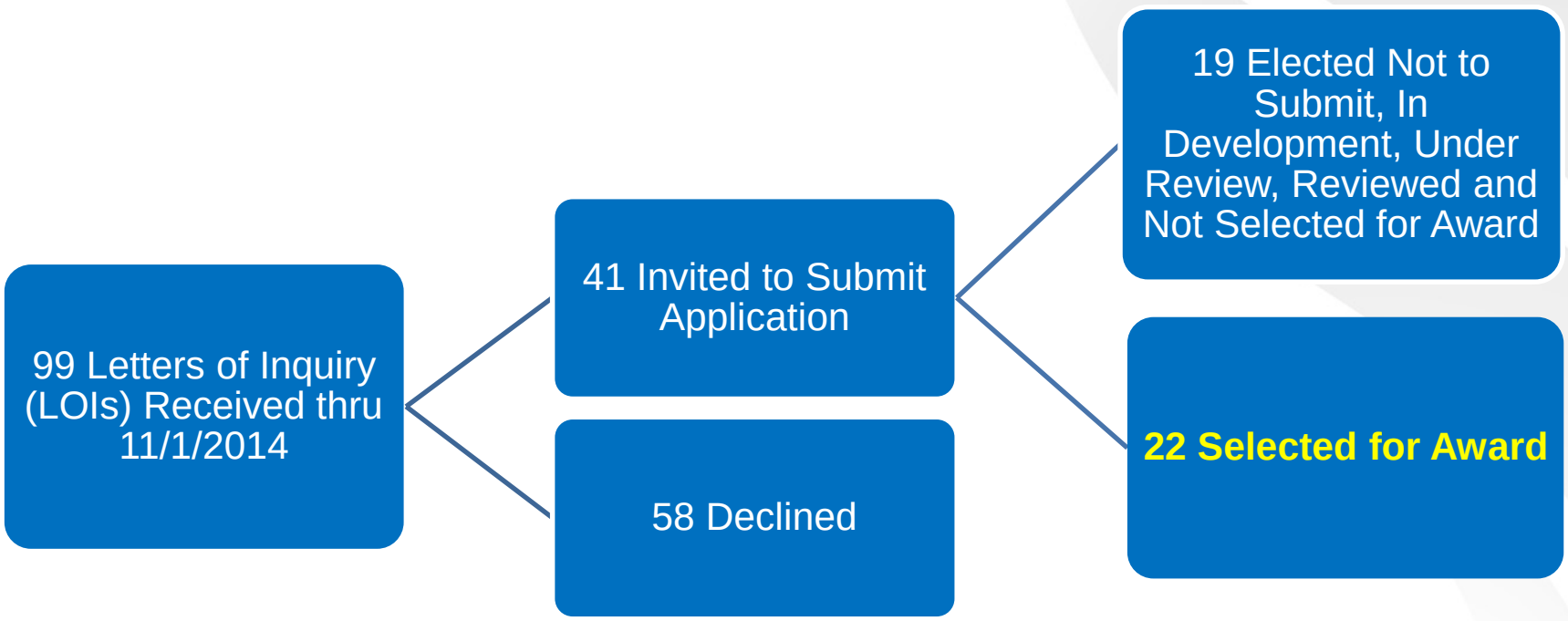
Engagement Awards: *Knowledge, Training & Development, Dissemination Awards**



*through November 1, 2014

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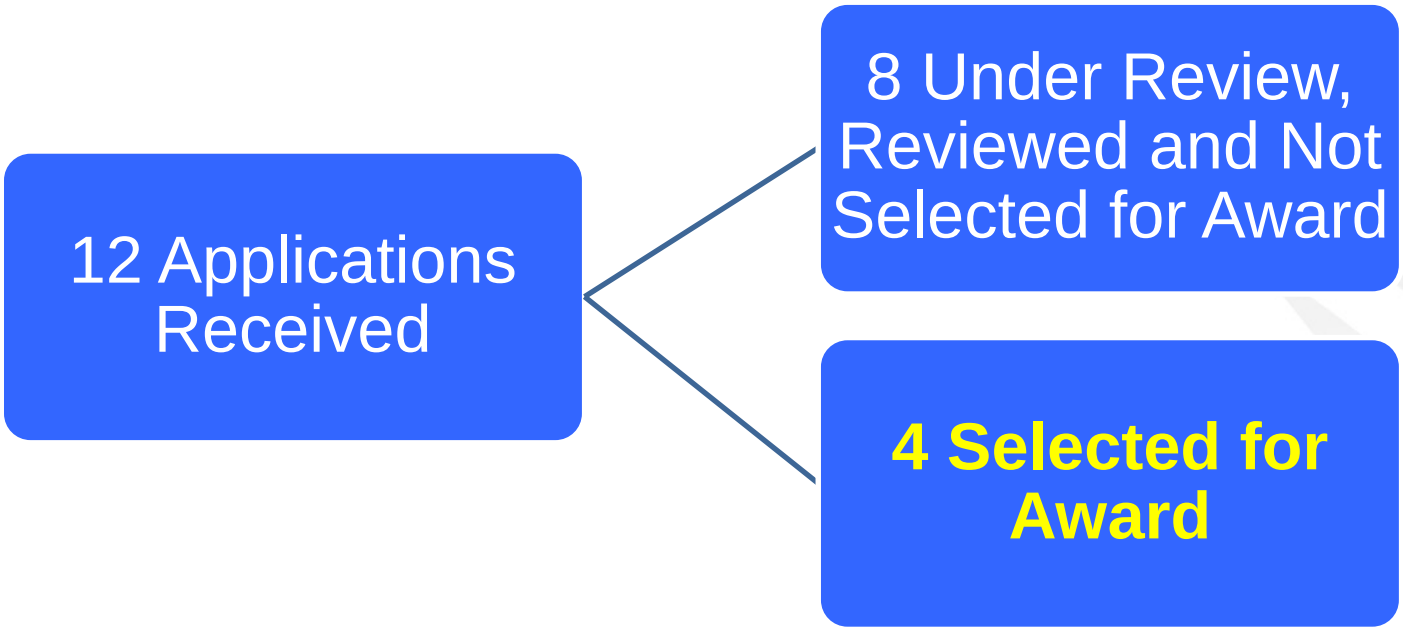
Engagement Awards: *PCOR/CER Meeting and Conference Support Awards**



*through November 1, 2014

Patient-Centered Outcomes Research Institute

Engagement Awards: *Looking Ahead*

Applications received

- 26 Projects Selected for Award
- 20 Awards Made (6 Pending Final Execution) totaling ~$4.0 M
- 6 in Queue for Award totaling ~$1.4 M

Growing application pipeline

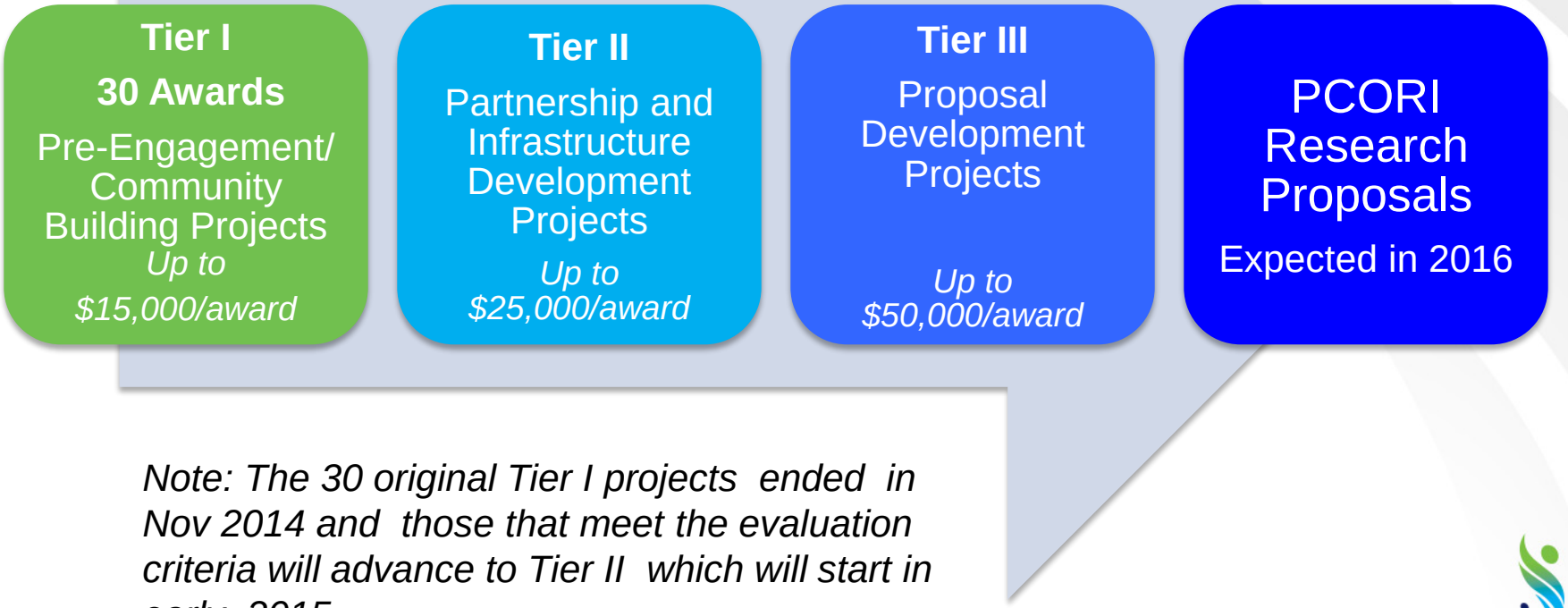
- 5 currently under review ($1.1 M)
- 10 applications in development ($2.2 M)
- Next review meeting in January

Program evaluation framework and metric development – in process

Pipeline to Proposal Awards

Where have we been?

Pipeline to Proposal Awards – Q4 Snapshot



Note: The 30 original Tier I projects ended in Nov 2014 and those that meet the evaluation criteria will advance to Tier II which will start in early 2015.

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Status of the Pipeline to Proposals Program Now

- **Refining the Pipeline Program:**

  Fine-tuning the Pipeline to Proposal Awards Program and training based on feedback from Pipelines Conference (11/7) and data from final reports on initial 30 awardees and first Program Office

- **Strengthening the Pipeline Program and Building a National Infrastructure:**

  Contracted with 4 additional Pipeline Award Program Offices for a total of 5 Pipeline Award Program Offices

- **Expanding the Pipeline Program:**

  Transition up to 30 Tier I Cycle 1 Pipelines to Tier II (Early 2015)

  Launch of 50 new Tier I Cycle 2 Pipelines (November 2014)

  Launch of 50 new Tier III Cycle 1 Pipelines (Early 2015)

The Future of Pipeline to Proposal Awards

- Continue the evaluation of and strategic planning
- Align program with PCORI's future research funding priorities by actively engaging Science in the Program's direction
- Structure awards to incorporate dissemination projects of PCORI-funded research projects or other evidence-based information aligned with PCORI's priorities

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The PCORI Ambassador Program

Ambassador Program Overview

The Ambassador Program is a volunteer initiative that organizes, trains, equips, and mobilizes patients, caregivers, organizations and other stakeholders to share PCORI's vision, mission and PCOR principles with their respective communities, participate as full partners in research and to help assure the sharing and uptake of information generated from PCORI funded projects.

Organizes: Provides community the opportunity to organize around affinity groups aligned with PCORI research priorities

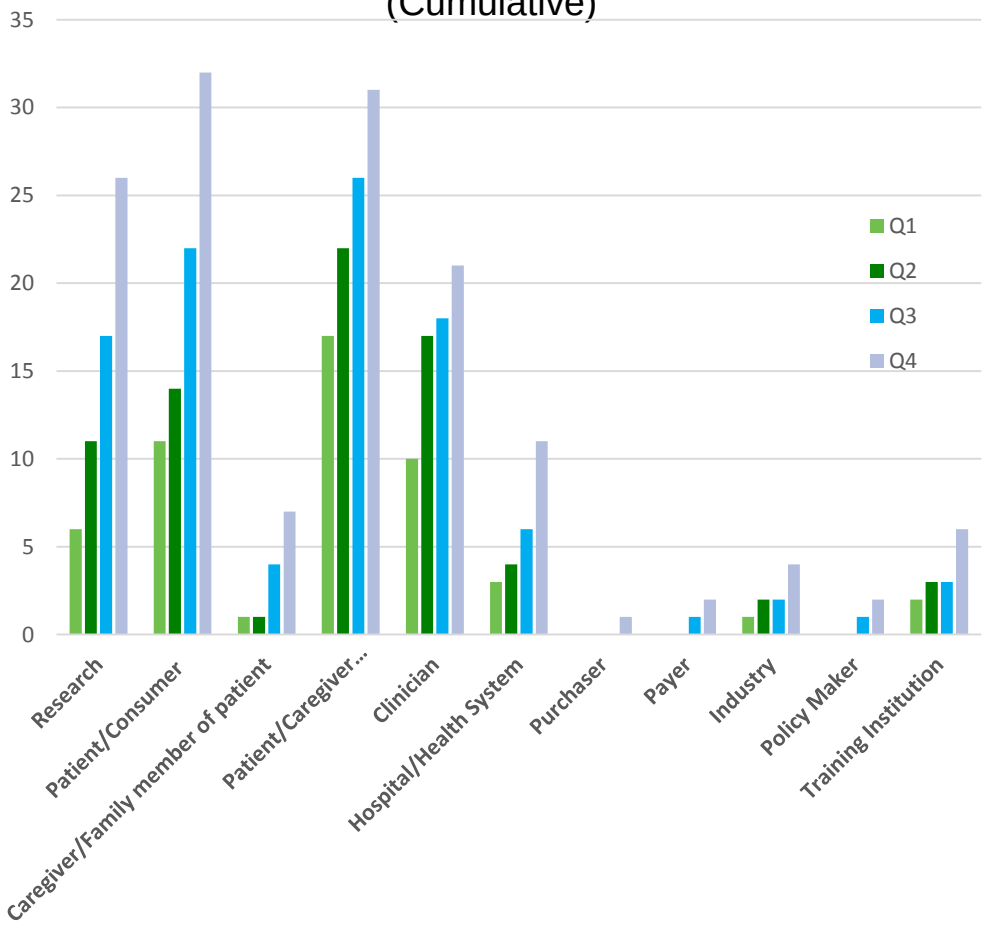
Trains: Informs community on PCOR/PCORI, patient-centeredness methodology standards, Engagement Rubric, PCORI funding, working in research teams and other more advanced training needs such as methodology, research design, etc.

Equips: Provides support material via a toolkit such as talking points, presentation template, and social media guide

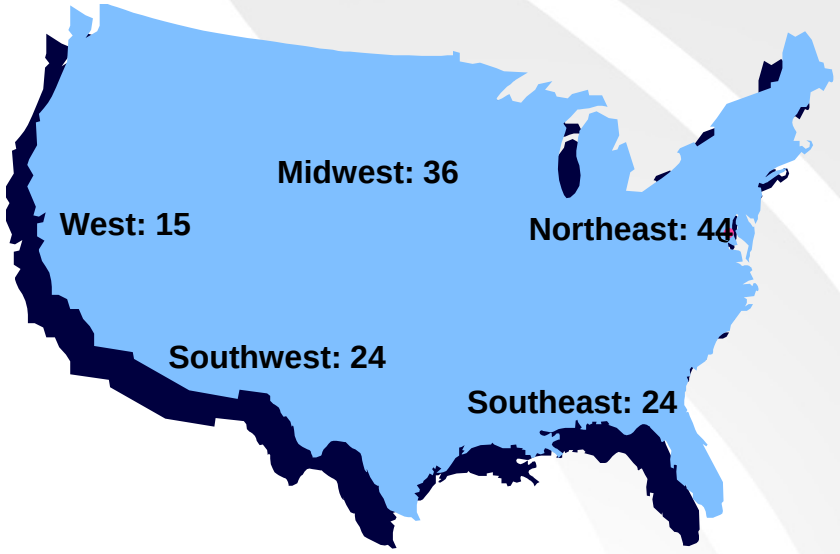
Mobilizes: Offers an Ambassador online community that encourages collaboration and the exchange of best practices and perspectives between different groups

PCORI Ambassador Program: Where We Are Now

Ambassadors Enrolled by Stakeholder Group
(Cumulative)



Ambassadors Enrolled by Geographic Region
(Cumulative)



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Training

Continuing Medical Education/Continuing Education Activities

- **Purpose: to provide certified professional educational activities that reflect PCORI's unique contributions to PCOR/CER**
  - Examples of proposed content:
    - Methodology Standards
    - Engagement Rubric
    - Communication and dissemination of research findings
- **Audiences:**
  - Primary care and specialty physicians
  - Physician assistants
  - Registered nurses and nurse practitioners
  - Pharmacists
  - Ambassador Program
  - Others, as identified

CME/CE Activities (continued)

- **Value Proposition:**
  - Support clinicians in maintaining professional excellence in medical, nursing, and pharmacy practice and patient care
  - Continue to strengthen the collaborative relationships we currently have with clinicians
  - Expand our reach to clinicians who may wish to become involved with PCOR/CER and PCORI
- **Currently negotiating with three contractors**
- **First Task Order: Methodology Standards**
  - Project time frame: mid-December, 2014 through April 30, 2015
  - Online and live activities
- **PCORI's 2015 Annual Conference:** research presentations

Training for External Stakeholders

- **Recently Completed:**
  - Merit Reviewer Training – Fall broad PFAs and Pragmatic Studies
  - Pipeline to Proposal: Applicants
  - Contracting with PCORI on Research Projects: Awardees
- **Projected:**
  - Awardee training for patient/stakeholder partners
  - Applicant and Merit Reviewer Training for 2015 funding cycles
  - Pipeline to Proposal: Awardees
  - Methodology 101

Strategic Questions for Board

Engagement Awards:

- As part of our ongoing analysis, we may begin to identify gaps in our portfolio
  - For example, we may find few awards focusing on dissemination, few awards to organizations in the Central US, few awards focusing on increasing engagement of policy makers, etc.

- Should we begin to be more directive and put out targeted announcements (i.e., Engagement Award Initiative Notices) calling for applications to address these gaps, or should we continue to allow ideas to grow organically?

- Should we put a cap on the amount/percentage of Engagement Award funds for PCOR/CER Meeting and Conference Support?

Strategic Questions for Board

General:

- As PCORI matures as an organization, what refinement of activities should we undertake to keep patient and other stakeholder engagement vibrant and meaningful?

- What are other ways to enhance integration of Engagement into our research portfolio?

Ways of Engaging- Engagement ACtivity Tool (WE-ENACT): Preliminary Results

Lori Frank, PhD

Program Director

Patient-Centered Outcomes Research Institute

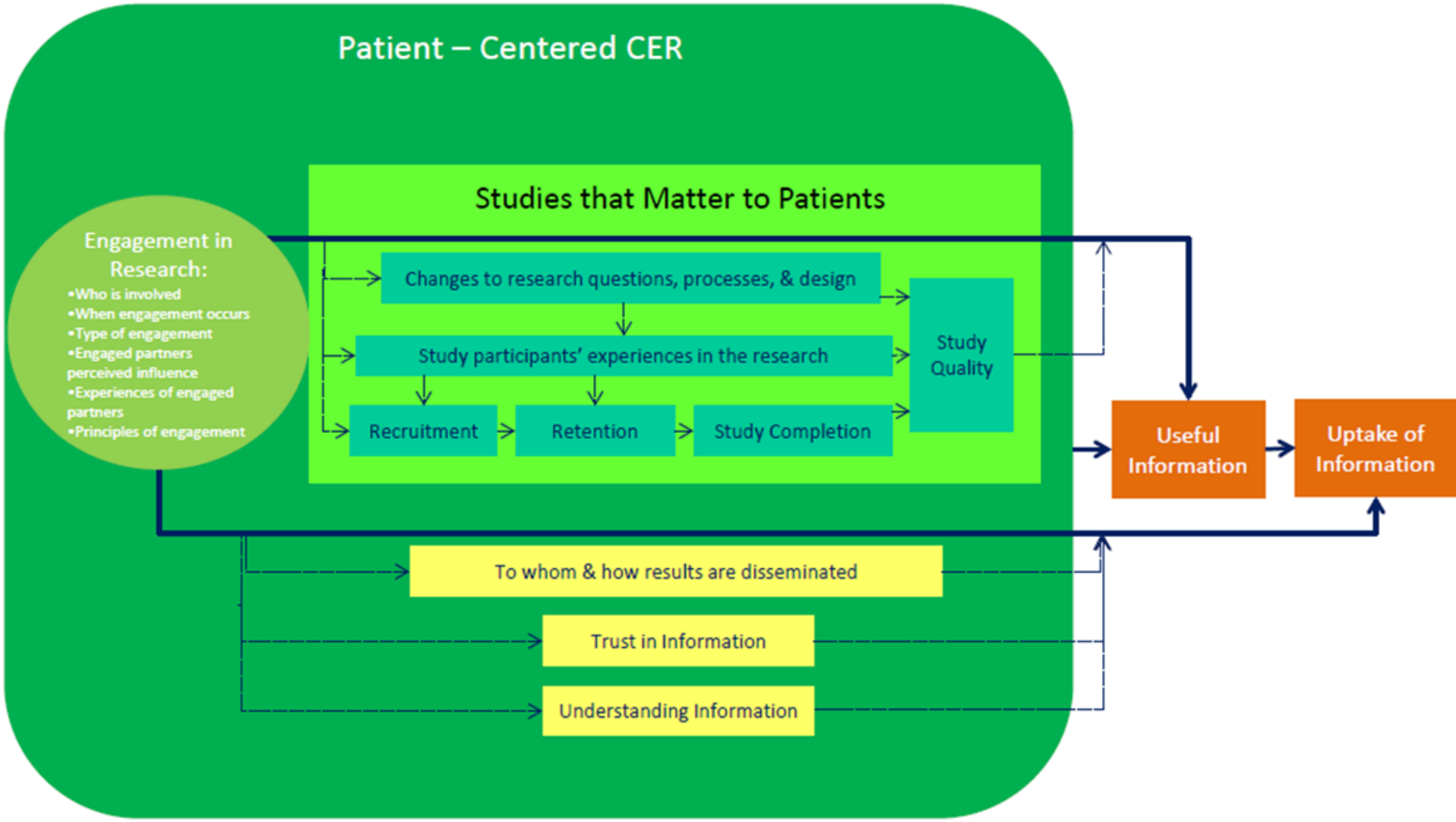
Multiple Objectives for Measuring Engagement

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

Domains for Describing Engagement in Research

- Who is engaged
- When they are engaged
- Partnership characteristics
- Level of research engagement
- Effects of engagement on research questions, processes, study design, implementation
- Perceived level of influence of partners
- Challenges, facilitators
- Lessons learned
- Evidence for PCOR principles

Evaluating Engagement in Research



Ways of Engaging - ENgagement ACtivity Tool: WE-ENACT

- Self-report
  - Principal Investigators
  - Patient and stakeholder partners
- Completed at baseline and annually
- Versions developed for
  - PCORI Pilot Projects
  - PCORnet projects
  - PCORI broad and targeted portfolio

Development of the WE-ENACT

Spring 2013

- Initial instrument developed by PCORI & Academy Health

July 2013

- Fielded with Pilot Project PIs

Fall 2013

- Engagement Rubric developed

Jan 2014

- Results shared with with Pilot Project awardees

Fall 2013 to Winter 2014

- Input from:
  - PCORI Evaluation Group (PEG)
  - PCORnet
  - Pipeline Program Office

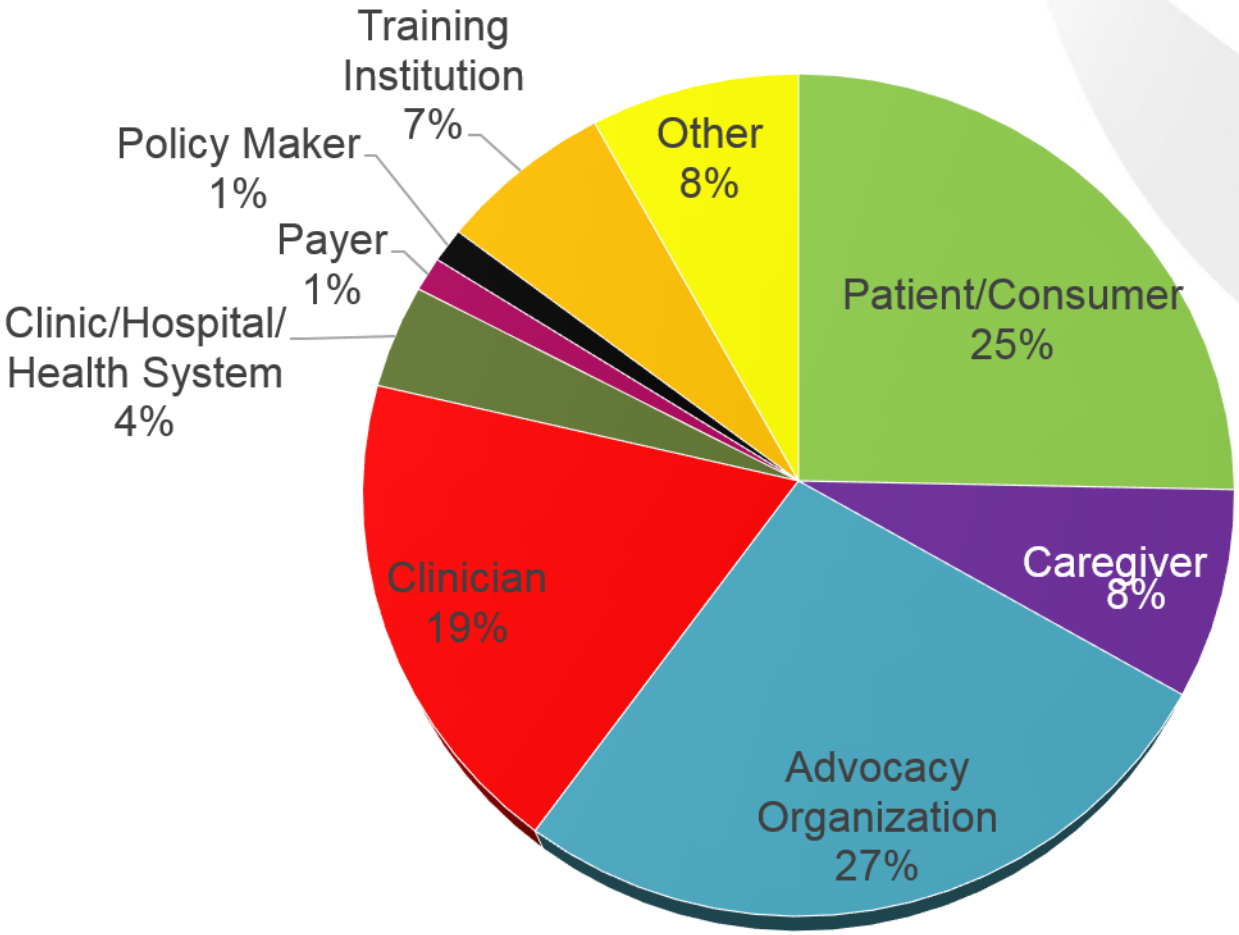
Summer 2014

- Reviewed by Patient Engagement Advisory Panel
- Cognitive testing

WE-ENACT: Preliminary Results

- Principal Investigators (PI) and patient and stakeholder partners from cycles I and II have been invited to respond to the 1 year inventory (n=70 projects)
- Current sample
  - 58 PIs or their designee *(data shown in blue)*
  - 75 patient or stakeholder partners, representing 29 projects *(data shown in red)*

Stakeholder Sample (n=75)



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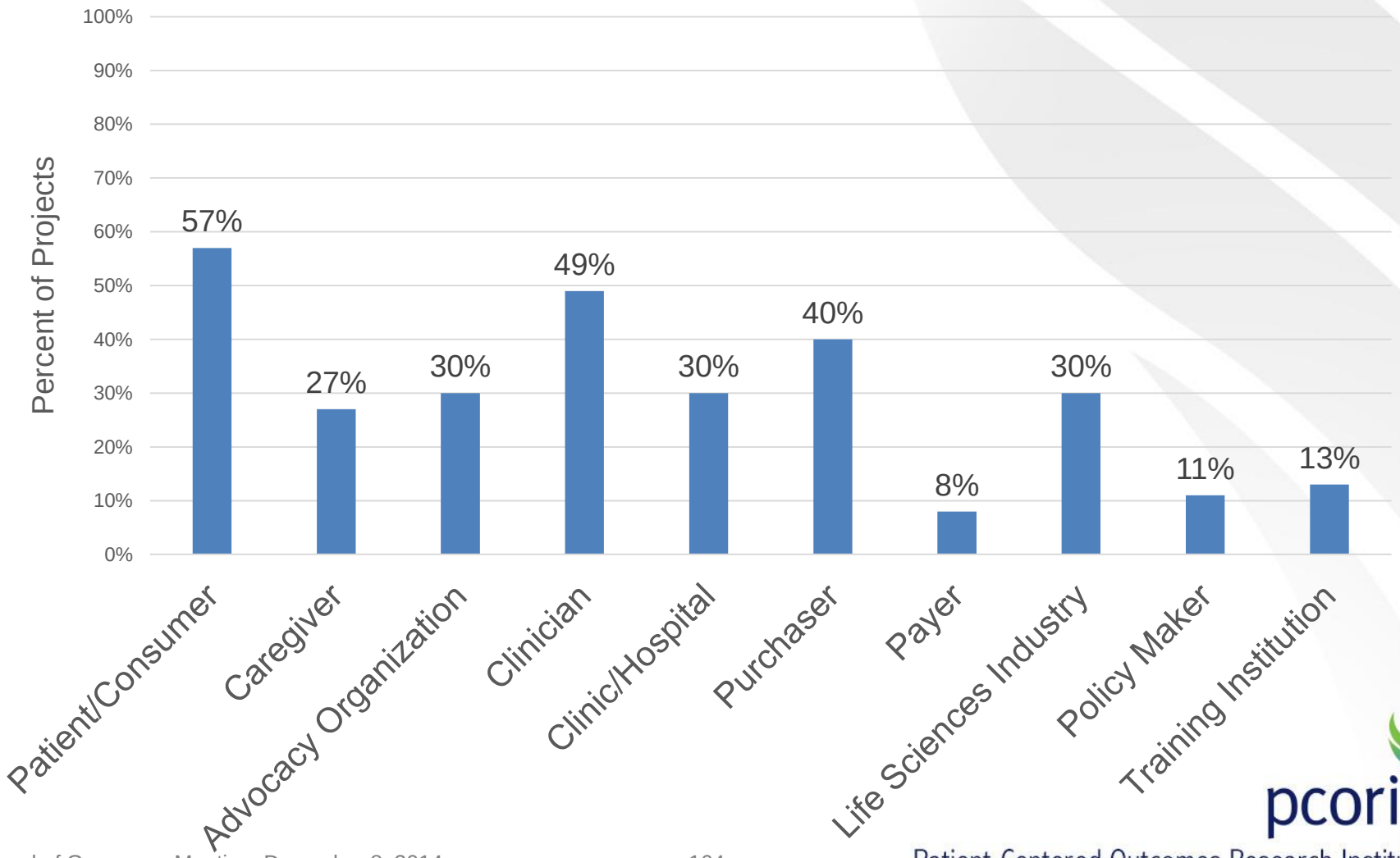
Types of Stakeholders Engaged
Researcher report



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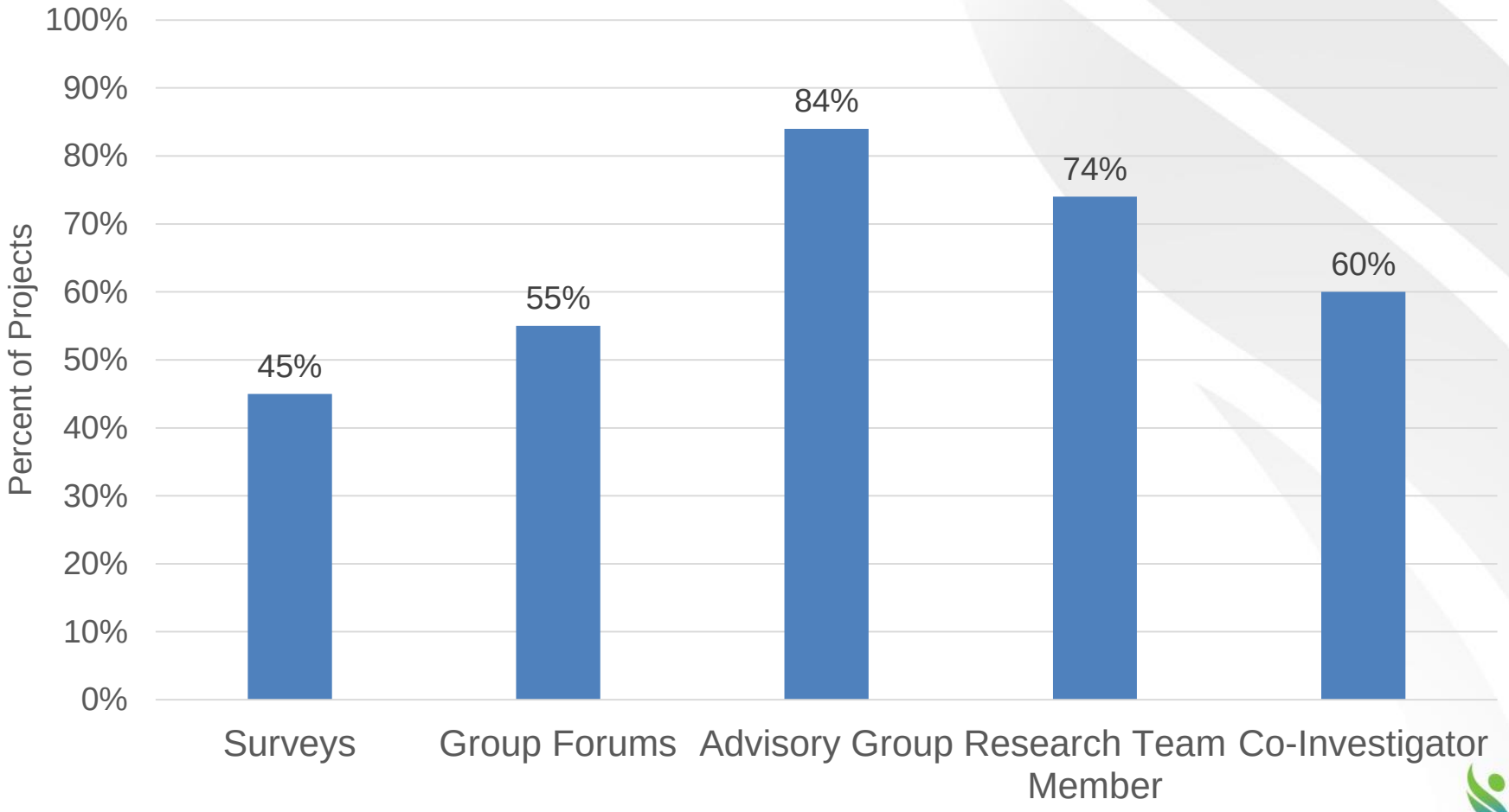
Approaches to Engagement
Researcher report



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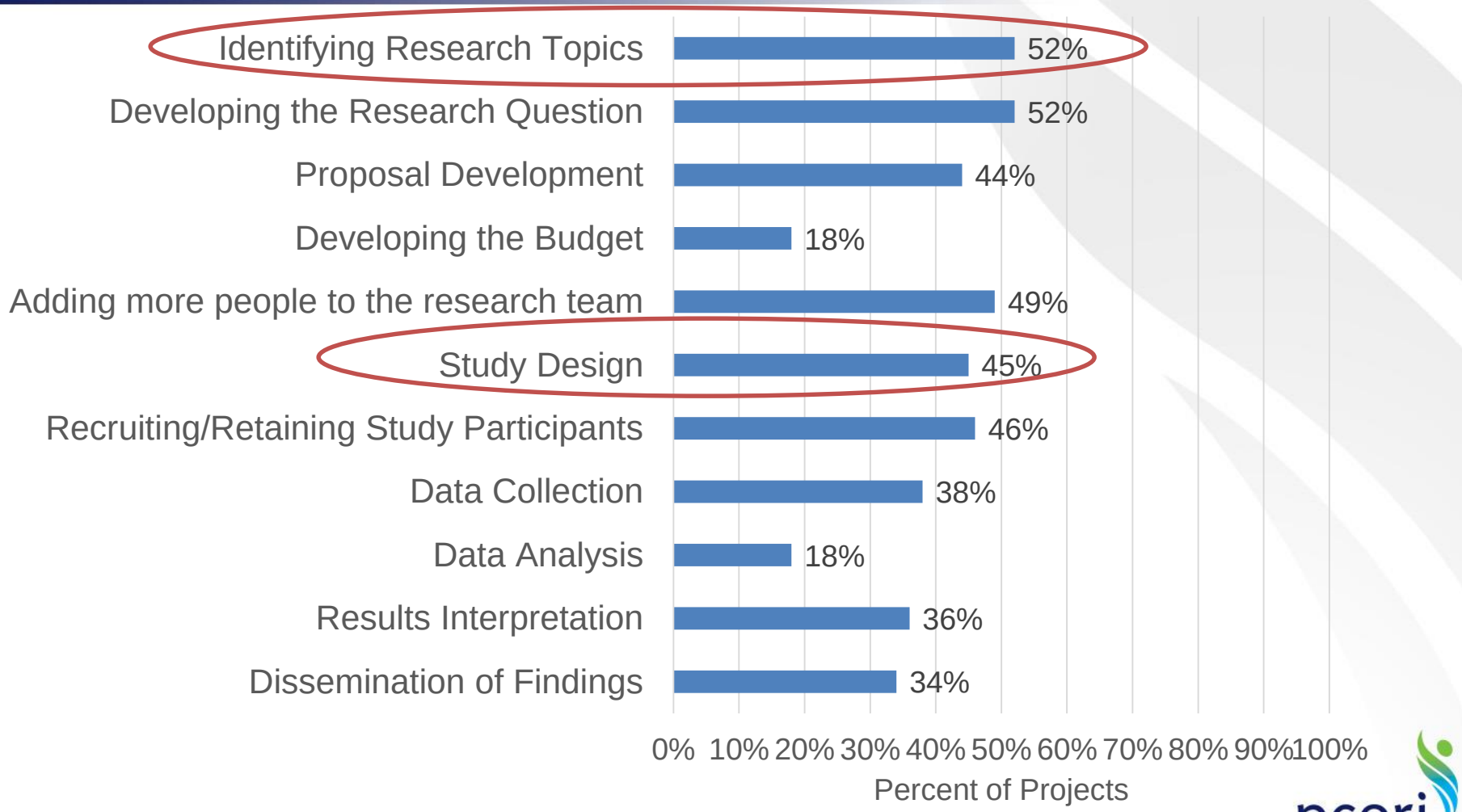
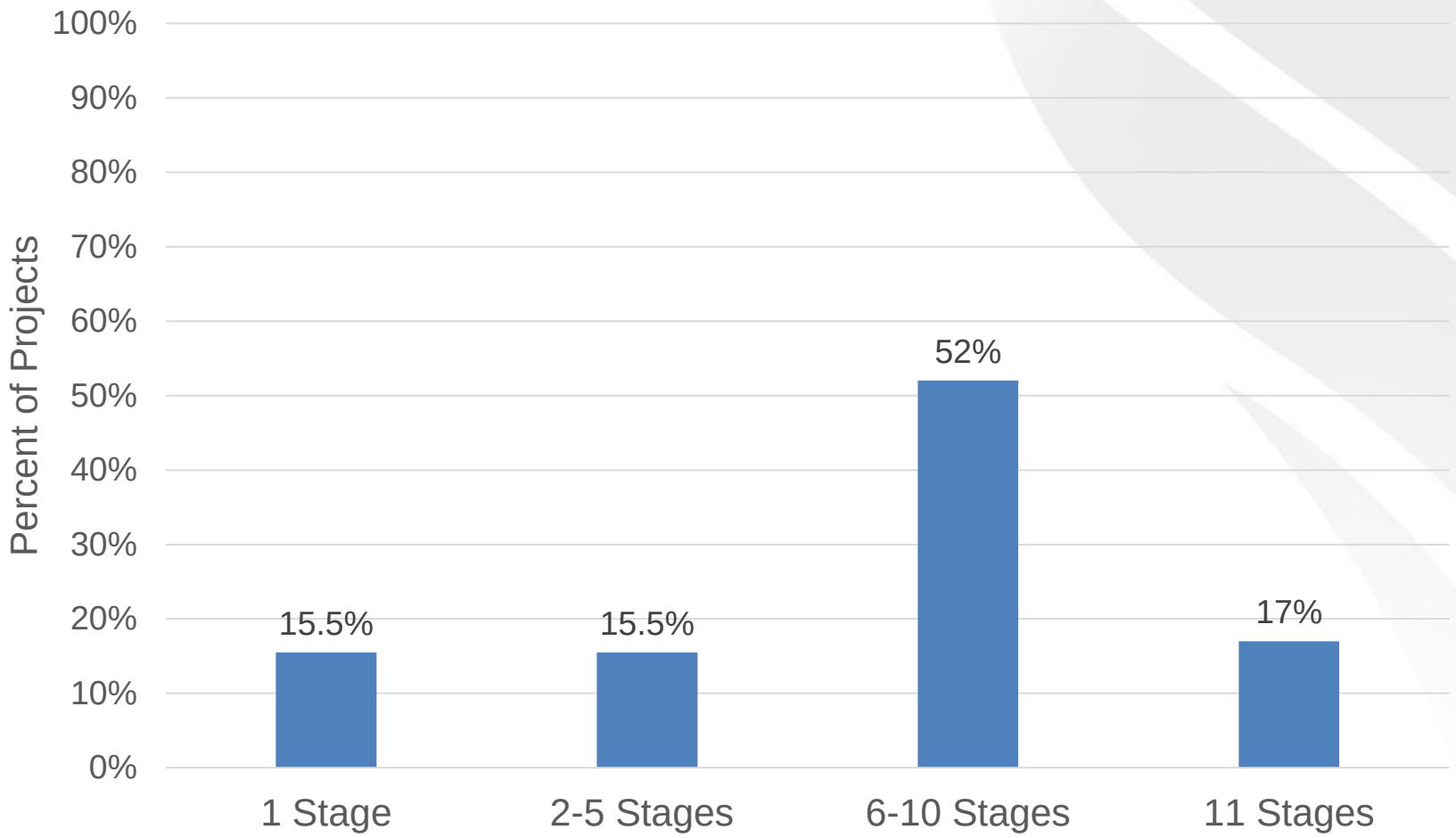
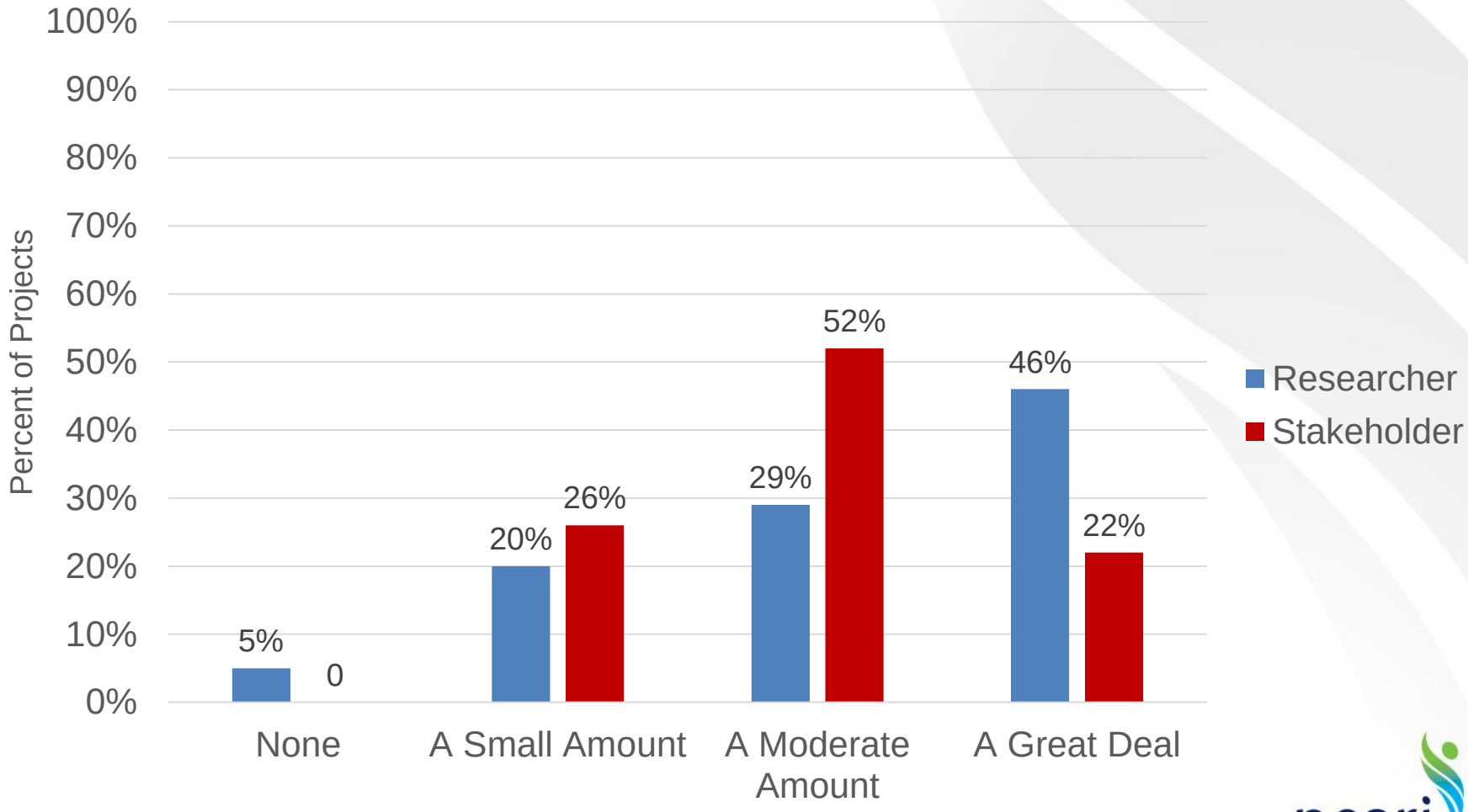
Research Stages with Engagement
Researcher report



Research Stages with Engagement
Researcher Report



Identifying Research Questions: Perceived Influence



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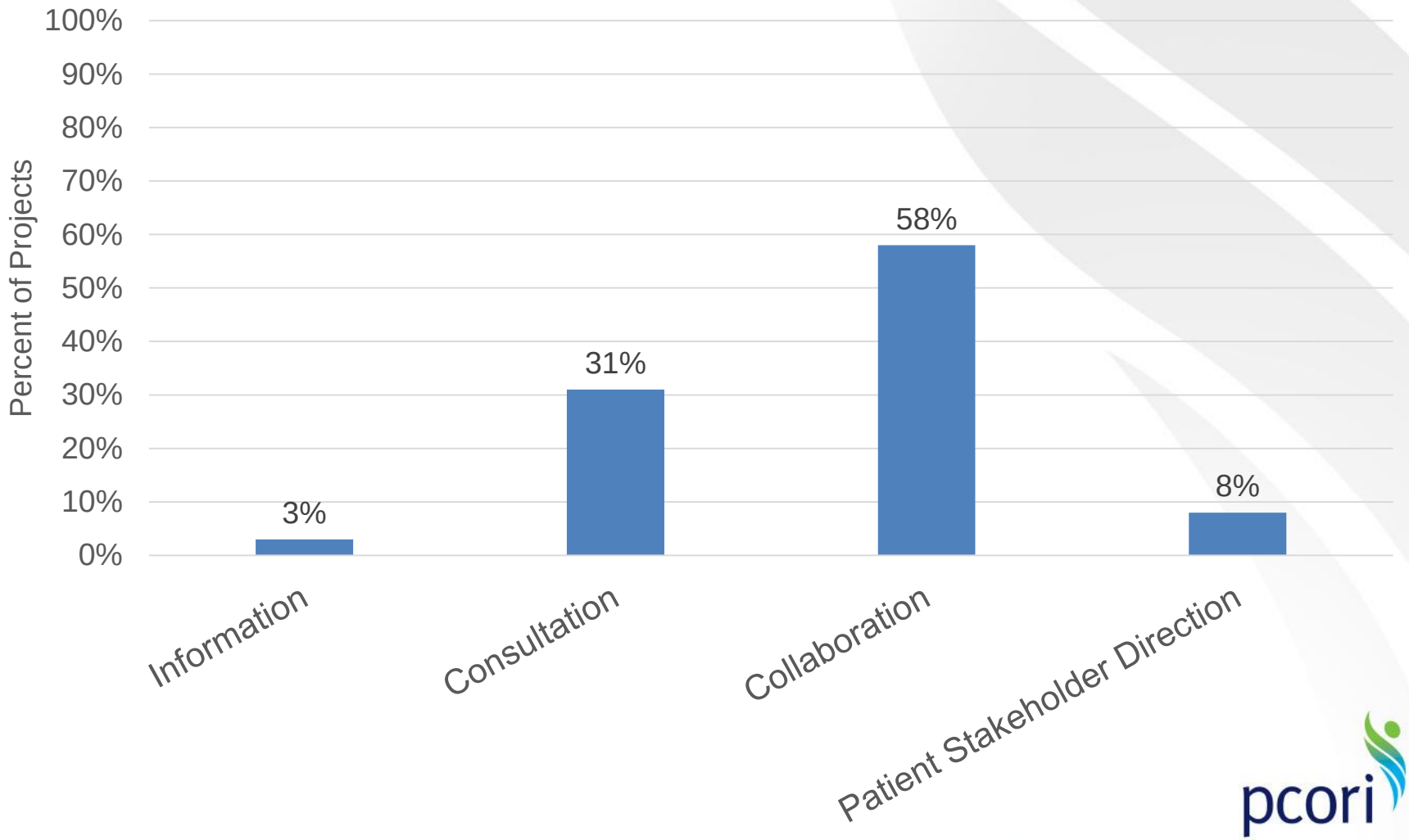
Identifying Research Questions: Impact of Research Engagement

"Their insight into the problem among patients in their community helped focus the research project."

"Topics were more tailored to parent and family concerns."

"Engagement with patients and other collaborative partners in identifying research topics ensures that research is exploring outcomes that matter to the individuals directly affected by the issue and the providers, policy makers who work with those individuals and have the power to influence practice based on findings."

Study Design: Level of Engagement
Researcher Report



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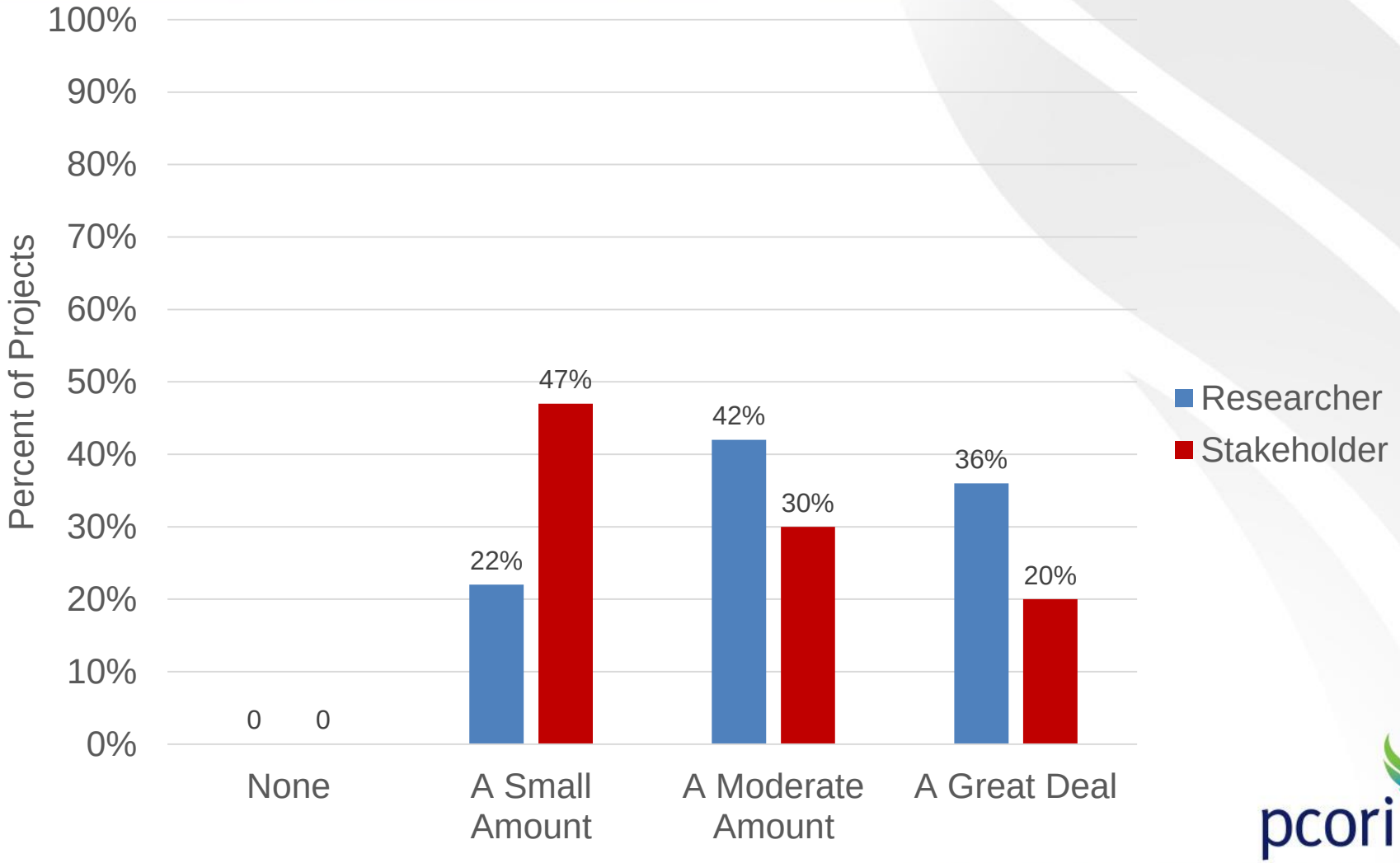
Study Design: Perceived Influence



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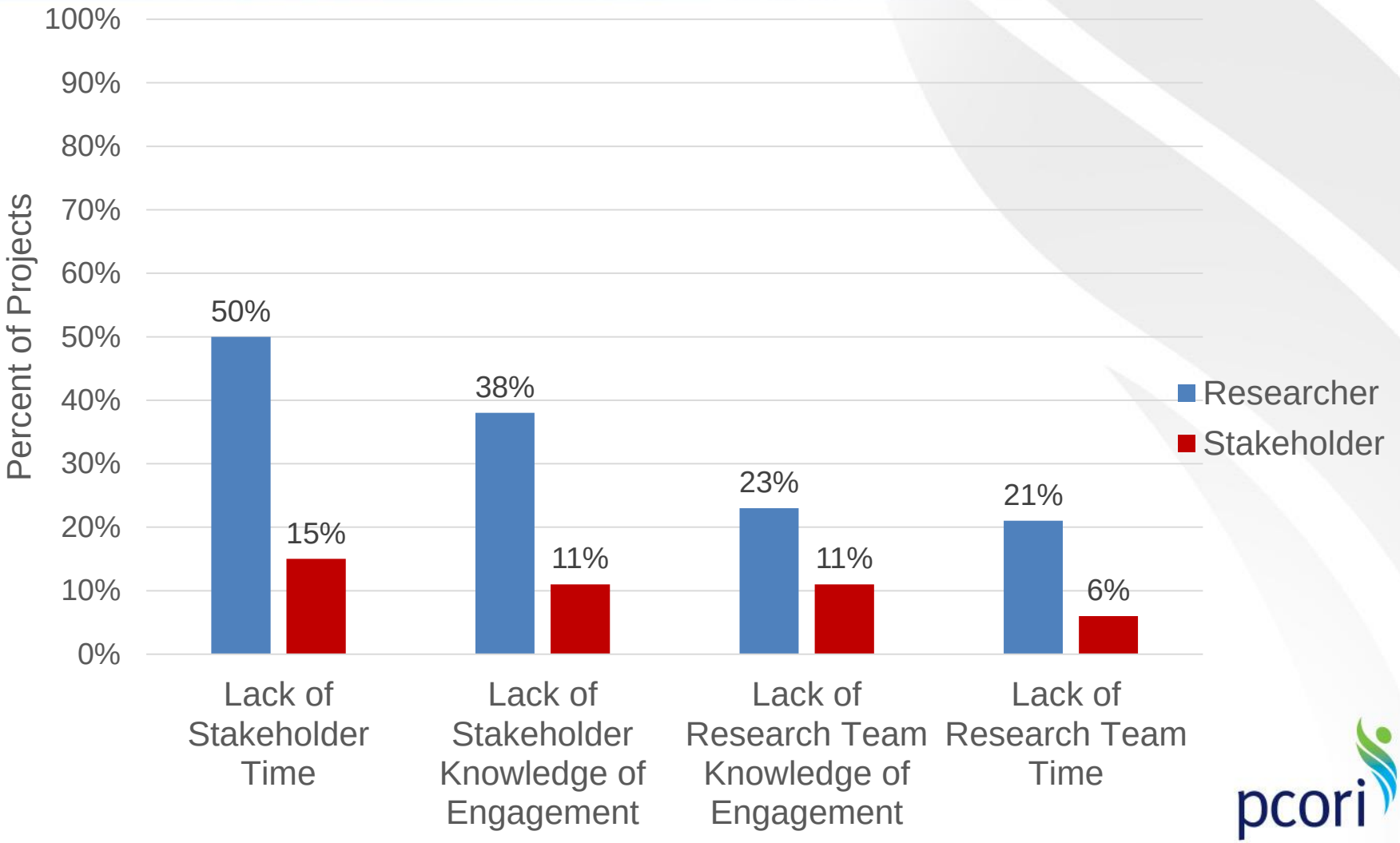
Study Design: Impact
Researcher Feedback

"Patients and stakeholders helped form the content of interventions… to better meet the needs of [patients]."

"Our community discussions… led to several modifications of our study design… This led us to include a 3rd group in our research design: community-based group exercise. We also decided to use… [a specific] outcome measure, based upon input from… patients who told us that their biggest concern was the ability to walk and stay active."

Engagement Challenges

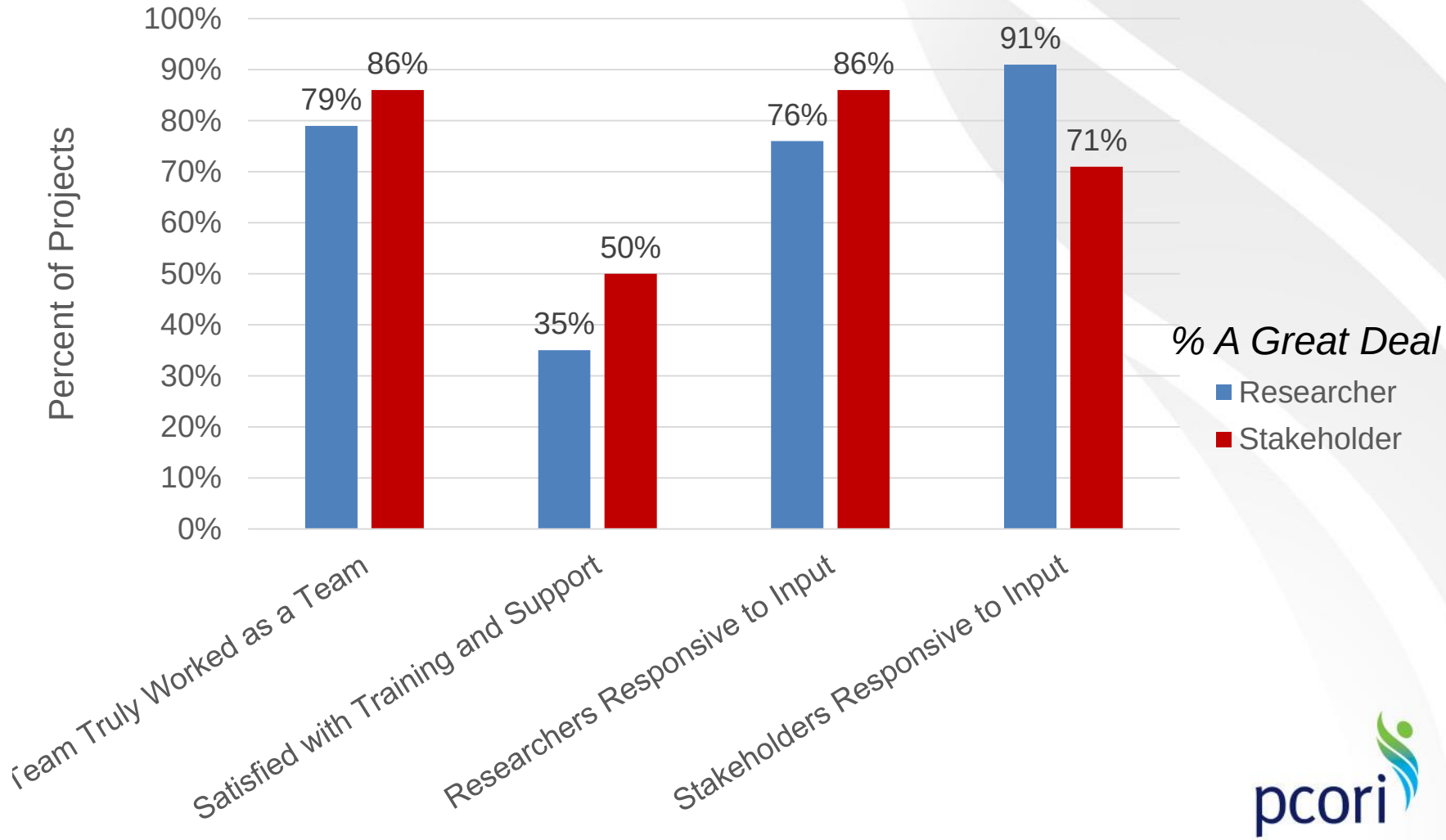


Engagement Challenges
Researcher Feedback

"Much more "face time" is required to build trust and learn about the culture you are going to. But the face time pays off."

"Some of our community partners are more outgoing than others; I have needed to work to ensure that all perspectives are elicited and heard."

PCOR Principles



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Patient and Stakeholder Feedback

"The researchers kept in very good contact with me, always answered my emails and always sent prompt updates on the project, I never wondered what was being worked on or what was needed from me, all data was shared with me, I felt very included in the team at all times."

"Was very impressed that this research team is open to discussion and took a lot of time and consideration in how the community wants to see some of the things they're doing. Very different than what has happened in the past. Institutions are opening up and valuing what the community has to say."

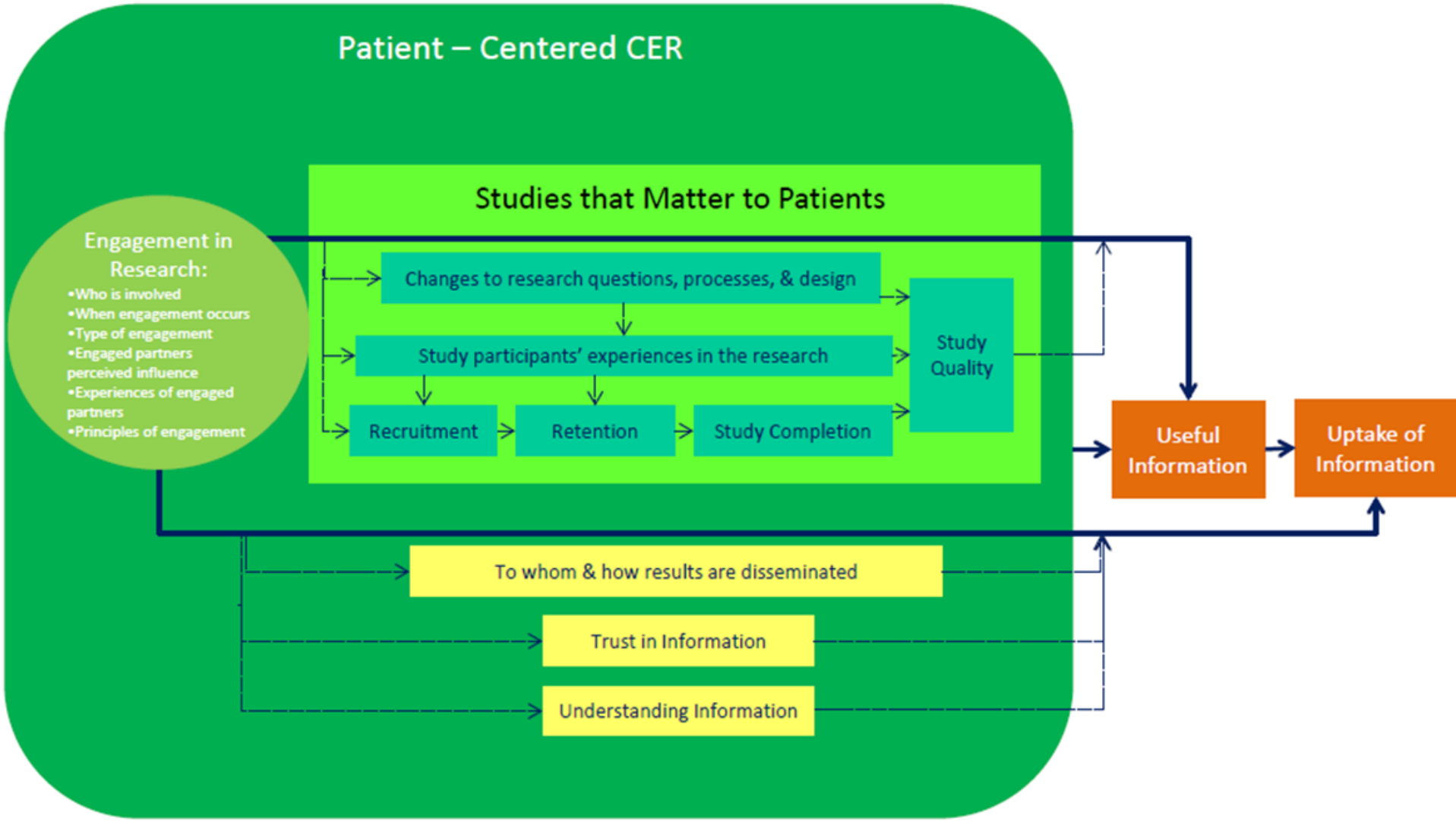
Summary

- PCORI awardees engage in research with a wide range of stakeholders, most often via advisory groups or as research team partners
- Engagement is occurring across all stages of research
- Perceived level of influence on research should be examined further to understand differences between research partners and principal investigators

For Discussion

- Which of the expected outcomes of engagement should be priorities for further examination?
- What are ideal targets for level of agreement between researchers and research partners for
  - perceptions of influence on research?
  - engagement barriers and facilitators?

Evaluating Engagement in Research



PUBLIC COMMENT

Join the conversation on Twitter via #PCORI

WRAP-UP & ADJOURN

Join the conversation on Twitter via #PCORI

Wrap-up and Adjournment

Grayson Norquist, MD, MSPH
Chair, Board of Governors

Patient-Centered Outcomes Research Institute