Patient Engagement Advisory Panel

October 1, 2014

Arlington, VA

Patient-Centered Outcomes Research Institute

Welcome, Introductions, and Review Agenda

Jean Slutsky, PA, MSPH
Chief Engagement and Dissemination Officer

Sue Sheridan, MIM, MBA
Director of Patient Engagement

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Agenda for Oct 1

9:00 – 9:30 a.m.	Welcome, Introductions and Review Agenda
9:30 - 11:15 a.m.	Rubric / Engagement Officers
11:15 a.m. – 12:15 p.m.	WE-ENACT Tool
12:15 – 12:30 p.m.	Group Photo
12:30 – 1:30 p.m.	LUNCH
1:30 – 2:30 p.m.	Introduction to Pragmatic Studies
2:30 – 3:30 p.m.	Update on Pipeline to Proposal Awards
3:30 – 3:45 p.m.	BREAK
3:45 – 5:15 p.m.	Update: Ambassador Program
5:15 – 5:30p.m.	Wrap-up
5:30 – 6:00 p.m.	BREAK
6:00 – 7:30 p.m.	Dinner with Advisory Panel on Improving Healthcare Systems

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Advisory Panel on Patient Engagement

Welcome!

Amy	Kratchman
Kimberly	McCleary
Charlotte	Collins
Stephen	Arcona
Paul	Arthur
Steven	Blum
Marc	Boutin
Kristin	Carman
Perry	Cohen
Amy	Gibson
Regina	Greer-Smith
Bruce	Hanson
Lorraine	Johnson
Julie	Moretz
Melanie	Nix
Sally	Okun
Laurel	Pracht
Darius	Tandon
Sara	van Geertruyden
Saul	Weingart
Leana	Wen

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Refining and Revising the Rubric

Sue Sheridan, MIM, MBA
Director of Patient Engagement

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Implementation

- Embedded in PFA materials for February 2014 funding announcements
- Provided as tool for merit reviewers
- Training provided through town halls and other mechanisms
- Initial feedback from applicants and merit reviewers
    - Valuable resource
    - Not perfectly aligned with the engagement portion of application
    - Would be helpful to have examples of stakeholder engagement

Revisions

- For August 2014 funding announcements
  - Revised format for greater clarity
  - Aligned rubric to mirror engagement portion of application, revised engagement portion of application to mirror rubric
  - Included stakeholder engagement examples (change from *Patient and Family Engagement Rubric* to *Engagement Rubric*)
- Options for the future
  - Methods-specific rubric
  - Pragmatic trials-specific rubric
  - Other tailored rubrics?

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Rubric as Catalyst

- Incorporating the rubric into the funding materials was the bridge for connecting engagement to the PFA development and revision process
- After the initial incorporation of the rubric, additional modifications were made, including:
  - The creation of a patient/stakeholder partner biosketch
  - Addition of language about engagement-specific milestones
  - The practice of publicly identifying the primary patient and stakeholder partners on a project in addition to the investigator

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Putting the Rubric to Work

- Inform awardees of availability of rubric (majority of current awardees were funded prior to the inclusion of the rubric in funding materials)
- On calls or in other correspondence, cite examples from the rubric for guidance
- As examples of engagement continue to surface, we can add to or revise the rubric

Sharing the Rubric

- Significant interest in the rubric from funders, patient and stakeholder advocacy groups, and researchers
- We continue to identify avenues for publication and sharing the rubric
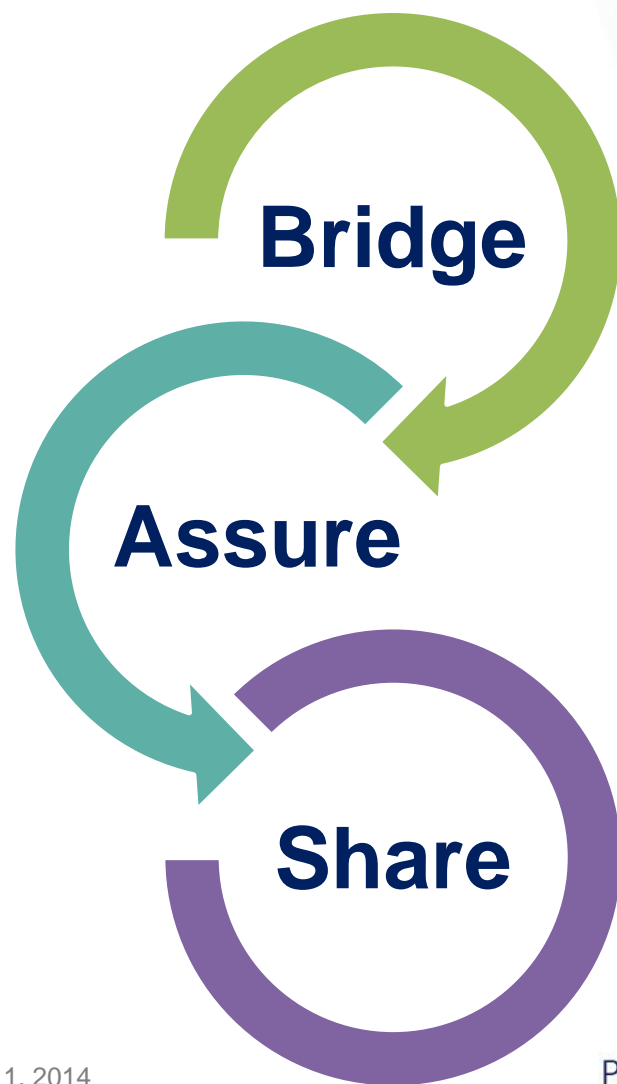- We have participated in multiple conferences and meetings to share the rubric and feature funded projects

The Engagement Officer Role: Experience to Date

Kim Bailey, MS
Engagement Officer

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Engagement Officers: Ensuring Research Done Differently

The Engagement Officer Role: Bridge

- Bringing Science and Engagement together; Connecting awardees, resources, and communities
- Inside PCORI
  - Act as a conduit between teams
  - Identify needs and possibilities
- PCORI projects
  - Share PCORI vision for engagement in research
  - Find common goals and shared interests

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The Engagement Officer Role: Assure

- Evaluating, monitoring, and augmenting engagement
- Inside PCORI
  - Identify trends, themes, and models of engagement
  - Highlight potential areas of alignment and opportunities for improvement
- PCORI projects
  - Evaluate and cultivate engagement
  - Monitor and troubleshoot challenges

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The Engagement Officer Role: Share

- Helping to tell the portfolio's story
- Replicating what works
- Inside PCORI
  - Refine funding announcements and processes
  - Aid evaluation efforts
- PCORI Projects
  - Highlight successes
  - Create (formal and informal) learning networks

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The Project Life Cycle

Merit Review

Pre-Award

Post-Award

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Potential Roles & Responsibilities

Merit Review

Merit Review

- LOI review
- Observation of review
- Slate selection

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Potential Roles & Responsibilities

Merit Review

Pre-Award

- Review and modify milestones

- Ensure adherence to Methodology Standards

- Augment engagement

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Potential Roles & Responsibilities

Merit Review

Post-Award

- Award letter

- Kick-off call

- Interim report review

- Interim calls

- Site visits

- Patient and stakeholder interviews

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Successes to Date

- Demand for Engagement Officer support
    - Inside PCORI
    - From awardees
- Interest in PCORI model of engagement and Engagement Officer role
- Projects improved
- Stellar projects highlighted and best practices shared

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Seeking Your Wisdom

- How should we evaluate the success of the Engagement Officer role?

- What metrics should we use?

- Are there existing models of program evaluation that we should consider using?

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Engagement in PCORI's Research Portfolio

Ayodola Anise, MHS
Program Officer

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Overview

- PCORI Evidence to Action Networks
- Engagement in the Addressing Disparities program asthma portfolio

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PCORI Evidence to Action Networks

PCORI Evidence to Action Networks

- PCORI is launching Evidence to Action Networks—learning networks with more engagement of end users
- Goals of networks are to:
  - Engage awardees, including patients and stakeholders, and facilitate cross-learning between funded projects **across PCORI**
  - Link awardees with end users (e.g., payers, employers, policy makers) to enhance relevance of evidence and increase likelihood of uptake of findings
- Networks can be organized around:
  - Health topic or condition
  - Methodology
  - Intervention

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Scope of Work for Evidence to Action Networks

- Selected contractors will perform a variety of activities to develop and implement networks, including:
  - Conduct surveys of awardees and end users
  - Conduct literature reviews on potential topics
  - Develop protocol on and implement methods and techniques aimed at facilitating communication and engagement
  - Evaluate the networks
- Network components could include:
  - Share-and-learn sessions
  - Creative dissemination strategies (e.g., TED-like talks, use of social media)
  - Technical assistance on various topics (e.g., writing a manuscript)
  - Online discussion forums

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Asthma Evidence to Action Network

- PCORI's first Evidence to Action Network will comprise asthma-related projects, including:
  - Addressing Disparities targeted asthma portfolio
  - Other asthma projects across programs, including projects in the IHS and Engagement portfolios

- First activity is a needs assessment to:
  - Understand needs, challenges, and strengths of research teams
  - Assess readiness to participate in network and collaborate with other awardees
  - Identify areas for collaboration

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Asthma Portfolio Update: Learnings on Patient and Stakeholder Engagement

…On Engagement

"We are very pleased with the level of engagement of our partners, and are grateful for the tremendous feedback and input that the [Stakeholder Engagement Core] members have provided. This has impacted our thinking in a number of key ways as we refine our proposed study design and intervention components."

Quote from Asthma Awardee Interim Report

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Background on Asthma Portfolio

- The Addressing Disparities program funded 8 projects in December 2013 through the Asthma PFA that:
  - Focus on reducing adverse outcomes due to poorly controlled asthma in African-American and/or Hispanic/Latino individuals, populations, and subgroups
  - Include patient-centered outcomes tailored to the needs of individuals and populations
  - Compare interventions to improve clinician and patient adherence to guidelines by:
    - Enhancing provider and patient communication (e.g., use of mobile technology, education).
    - Improving systems of care (e.g., evaluate models integrating schools, home, and clinic, as well as EHRs).
    - Improving integration of care (e.g., team-based care, CHWs).

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Asthma Portfolio: Built on Engagement

- Interventions are multi-level, multi-component, and some include community-level components
- Projects are patient-centered and include strong patient and stakeholder partnerships and engagement
- Projects make use of 2-stage approach to project implementation
  - Stage 1 activities focus heavily on engagement and building partnerships
    - Activities include developing materials and protocols, conducting focus groups, tailoring educational tools, and obtaining clearances from all institutional and community partners.
  - Stage 2 activities focus on refining, implementing, and evaluating the interventions

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Shared Learnings on Patient and Stakeholder Engagement

◉ In July, program staff convened two webinars with the eight asthma project teams to discuss patient and stakeholder engagement, including:

- Successes, challenges, and lessons learned
- Engagement strategies with respect to study design, implementation, and dissemination

◉ Awardee presentations were framed using the Engagement Rubric

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Overall Engagement Themes

Shared success factors and successful activities:

Focus Groups and Interviews	Patient and Stakeholder Advisory Boards	Comprehensive and Coordinated Engagement
• Obtaining information from patients, clinicians, CHWs on key features of study and intervention	• Obtaining detailed feedback on all aspects of the study including study design and implementation • Providing "reality check"	• Obtaining input from patients with asthma including teens, where appropriate • Involving payers, key personnel at clinics, public health, housing, state legislature, etc.

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…On Engagement
Focus Groups and Interviews

"Throughout the focus groups, patients reported that their asthma is well controlled, but also state that they use their rescue inhaler daily, indicating this may not be well controlled. This finding was informative for the design of EMR prompts for physicians. The prompt will be designed to ask about specific symptoms, rather than simply asking how a patient's asthma is."

Quote from Asthma Awardee Interim Report

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…On Engagement
Patient and Stakeholder Advisory Boards

"The [Stakeholder Engagement Core (SEC)] discussions have directly informed our current round of qualitative research. The discussion guide includes topics that arose during the SEC meetings, and addresses some of the areas where individual SEC members either provided divergent perspectives, or shared poignant experiences that we want to explore further. We anticipate that the SEC members will help us interpret [data from focus groups and one-on-one interviews] and further translate the findings of the qualitative research into concrete intervention components and patient-centered outcomes."

Quote from Asthma Awardee Interim Report

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…On Engagement
Comprehensive, Coordinated Engagement

"We have assembled a diverse team of over 35 collaborators and stakeholders from academic medical centers, key state and city government agencies, community-based social and health service providers, payers, and a non-profit hospital advocacy organization."

Quote from Asthma Awardee Interim Report

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Overall Engagement Themes (cont.)

Shared challenges with respect to three main areas:

Implementation	Analysis	Dissemination
• **How best to engage stakeholders, especially patients, in collecting data and monitoring the study?**	• **How to engage patients in interpretation of data?** • **How do we get help from stakeholders to tell the story?**	• **How to involve patients in dissemination (e.g., during study, post-study, in communicating/ translating findings)?**

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Immediate Requests

Using of social media

Engaging youth

Engaging community doctors

Leveling the playing field

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Next Steps

- **Research teams encouraged to**:
  - Reach out to each other to offer help, seek help, or brainstorm
  - 4 teams with CHW focus are working together to streamline protocols, collection tools, and timelines
- **PCORI staff will**:
  - Update Evidence to Action Network facilitator on learnings and input to date to inform initial activities of the network
  - Conduct site visits with goals of:
    - Better understanding best/promising practices with engagement and the conduct of patient-centered CER and challenges and solutions to overcome challenges
    - Telling the story of the process, progress, and results of a project

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Evaluating the PCORI Way:
Measuring Engagement in PCORI Funded Projects & Responding the PEAP Evaluation Recommendations

Laura Forsythe, PhD, MPH
Senior Program Officer for Research Integration and Evaluation

Kristen Konopka, MPH
Senior Program Associate for Stakeholder Engagement

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Multiple Purposes for Measuring Engagement

- **Describe** engagement in PCORI funded projects
  - Enhances communication with awardees, other researchers and potential research partners, external stakeholders
- **Evaluate** engagement in PCORI funded projects
  - Determine effect of engagement on PCORI strategic goals and other key outcomes of interest
  - Inform Engagement Rubric
  - Identify best practices for Engagement
  - Inform PCORI funding requirements for engagement

Challenges for Measuring Engagement in Research

- Engagement in research is a relatively new concept
- Limited evidence base
- Complex set of behaviors
- Subjective
- Dynamic

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Domains of Interest for Describing Engagement in Research

- Who is engaged
- Partnership characteristics- how formed, length, frequency of engagement, etc.
- Level of research engagement
- Which phases of research
- Effects of engagement on research questions, processes, study design, implementation
- Perceived level of influence of partners
- Challenges, facilitators
- Lessons learned
- PCOR principles (respect, co-learning, etc.)

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Measuring impact: *Changes to research questions, process, and study design*

- Perceptions of scientists and patient and stakeholder partners provided via the WE-ENACT
- Perceived level of impact on:
  - each stage of the research process (question framing, study design, recruitment, etc.)
  - the way researchers and partners work together on this project
  - research projects, other work, and relationships outside this specific project
- Description of specific impacts of engagement on each of the above

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Ways of ENgaging ACtivity Tool: WE -ENACT

- Self-report information collection tool
  - Principal Investigators
  - Patient and stakeholder partners
- Field at baseline and 12, 24, and 36 months post-contract execution (for 3 year projects)
- Versions developed for:
  - PCORI Pilot Projects
  - PCORnet projects
  - PCORI broad and targeted portfolio

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Development of the WE-ENACT

Spring 2013

- Conceptual model of PCOR reviewed by PEAP
- Initial tool developed by PCORI & AH

July 2013

- Tool fielded with Pilot Project PIs

Fall 2013

- Engagement Rubric developed

Jan 2014

- Webinar with Pilot Project awardees re: initial findings

Fall 2013 to Winter 2014

- Input from:
  - Pilot Projects
  - PCORI Evaluation Group (PEG)
  - PCORnet
  - Pipeline IF

Summer 2014

- Eval recommendations from PEAP
- Cognitive testing with PIs and partners

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Sample WE-ENACT items

- In which parts of the research project have you been engaged? *Select all that apply.*
  - Identifying research topics
  - Developing the research question
  - Proposal development
  - Developing the budget
  - Adding more people to the research team
  - Study design
  - Recruiting or retaining study participants
  - Data collection
  - Data analysis
  - Results review interpretation, or translation
  - Sharing study findings
  - Other *Please describe:*

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Sample WE-ENACT items

We want to learn about how you have influenced the research project. For example, did you contribute to decisions or processes from the project? Rate the amount of influence you had on each of the parts of the project listed below.

1. None
2. A Small Amount
3. A Moderate Amount
4. A great deal
5. I don't know whether I influenced this part

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Sample WE-ENACT items

- Please share the impacts you had on *<part of the project>.* What was changed or different because you were engaged in the research project?

- Please share anything about engaging with researchers on this project that you did not like.

- Please share anything you learned about how to engage patients and other stakeholders for *<part of the project>*?

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Sample WE-ENACT items

Please rate the following

1. Not at all
2. A Little
3. Somewhat
4. A great deal

- The researchers responded to input from stakeholders.
- The researchers, patients, and stakeholders on the team truly worked together.
- The research project is designed to address the needs of patients.

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How the Information Collected will be Used

- Review by Science and Engagement staff to understand project, discuss lessons learned about engagement, or help support the success of projects

- Identify promising approaches

- Provide guidance to current awardees, future applicants, and others interested in conducting or participating in PCOR

- Share with the public through presentations, publications, or other communications

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PEAP Evaluation Recommendations

PEAP Evaluation Recommendations

- April 29, 2014- PEAP Subcommittee on Evaluation presented their recommendations to PCORI for specific considerations around evaluation of our work

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Recommendations Included

- Highlight impact of *"what's the difference"* of doing research with engagement of patients and stakeholders

- Measure awardee attitudes toward engagement

- Communicate evaluation efforts in user- friendly language to the array of key PCORI stakeholders

- Track how PCORI's work influences others

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Timeline

May 2014	June 2014	June- August 2014	September 2014
PCORI Engagement Staff and Leadership Reviewed Recommendations	PCORI Evaluation Team Reviewed Recommendations	PCORI Evaluation Team Mapped Recommendations to current and planned evaluation work	PCORI Evaluation Team Drafted Response to PEAP Recommendations

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Mapping PEAP Recommendations to Evaluation Activities

- PCORI incorporated suggested language into the We-ENACT tool, a survey of funded researchers and patient/stakeholder partners on their practices and attitudes around engagement.

- PCORI has developed tools to track the impact of how we fund "research done differently" (ie; PCORI Evaluation Framework, merit reviewer & applicant survey work) as well as tools to measure the value of engagement in research we fund (e.g.; We-ENACT, net-ENACT).

- PCORI is collecting feedback from external stakeholder communities on their attitudes of PCORI and our work.

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Ongoing Evaluation Efforts

- The PCORI Evaluation Framework organizes all of the questions our stakeholders and PCORI staff have submitted about our work and outlines the questions we'll address and how we'll go about answering them. Many of the Subcommittee's suggestions are reflected in this strategic document.

- The PCORI Evaluation Group (PEG) is a panel of internal and external experts in evaluation and healthcare research that advises PCORI on evaluation efforts. Steve Blum represents the Advisory Panel on Patient Engagement on the PEG.

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PCORI Evaluation Activities

PCORI Evaluation Activities

Evaluation Program	Description
PCORI Evaluation Framework	The PCORI Evaluation Framework organizes all of the questions our stakeholders and PCORI staff have submitted about our work and outlines the questions we'll address and how we'll go about answering them. It includes questions about how we conduct our day to day work, how we are achieving our strategic goals, and ultimately, if and how this approach to "research done differently" will make a difference. Our framework is not static; it will continue to evolve along with our work because even as we attempt to answer the initial questions, new ones arise. We continue to welcome your feedback on the Framework.
Comment Opportunities	The PCORI evaluation team informs the public of new and ongoing evaluation work through public blogs. These blogs are about PCORI's evaluation activities and offer opportunities for external audiences to share feedback about our work.
PCORI Dashboard	PCORI utilizes a visual representation of metric tracking to serve as a primary mechanism for reporting on our programmatic progress to the Board of Governors. The Dashboard is now utilized to reflect outputs and ongoing processes that represent progress to our programmatic goals. The Dashboard is updated and presented to the Board in a public meeting on a quarterly basis.
Usefulness Criteria	PCORI has developed draft criteria to assess the potential usefulness of information from PCORI-funded studies. These have been presented to the Advisory Panels at the January 2014 meeting, the PEG, and has undergone internal testing.

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PCORI Evaluation Consultation

Evaluation Program	Description
Multi-stakeholder Advisory Groups	PCORI is governed and advised by a number of multi-stakeholder groups representing communities across the healthcare industry. These include the PCORI Board of Governors, the PCORI Evaluation Group, and the PCORI Advisory Panels. These bodies serve to guide and monitor PCORI's work to ensure adherence to the guiding mission and strategic goals of the organization.
PCORI Evaluation Group	The PCORI Evaluation Group (PEG) is a panel of internal and external experts in evaluation and healthcare research. The PEG is comprised of PCORI staff members, including representation from the Science and Engagement teams, members of the PCORI Methodology Committee, members of the PCORI Board of Governors, and external advisors. Steve Blum, member of the Advisory Panel on Patient Engagement serves as a representative on the PEG, and functional liaison between the two groups. The PEG provides targeted feedback on: • PCORI evaluation goals, • Methods for achieving those goals, and • Consultation on dissemination opportunities for results of PCORI program evaluation.

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PCORI Merit Review Evaluation

Evaluation Program	Description
PCORI Merit Reviewer Survey	PCORI surveys merit reviewers to obtain perspectives of the merit review process from scientist, patient, and other stakeholder reviewers; to improve internal merit review at PCORI; to contribute to the study of stakeholder inclusion in merit review.
Merit Reviewer Group Interviews	PCORI convenes focus groups of merit reviewers to obtain perspectives of merit reviewers on the merit review process; permits follow up on results from survey. Information is used to improve internal merit review processes at PCORI; information intended to contribute to the study of patient and other stakeholder inclusion in merit review.
Merit Reviewer Score Analysis	PCORI analysis the merit reviewer scores following a review session to understand the impact of PCORI inclusions of scientist, patients, and other stakeholders in review of health research funding applications. This assists in our effort to evaluate the impact on project quality, portfolio contents, participants and to improve the process as needed.
PCORI Funding Applicant Survey	PCORI surveys applicants to understand their experience of applying to PCORI for funding and to identify areas for improvement.

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PCORI Engagement Program Evaluation

Evaluation Program	Description
Engagement Event Survey Work	PCORI surveys participants of PCORI hosted events before and following every program. Surveys are issued Pre-Post, and 6 months following PCORI Workshops to track PCOR activities of workshop participants subsequent to events; to improve effectiveness of PCORI events.

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PCORI External Stakeholder Views on PCORI, CER, PCOR & Engagement in Research

Evaluation Program	Description
Survey of Patient and Clinician Views on CER and Engagement in Research	PCORI commissioned a survey with InCrowd to collect information on patient and primary care clinician attitudes and knowledge regarding comparative effectiveness research and engagement in research.
PCORI Clinician Survey	PCORI commissioned nationally representative surveys to collect information on attitudes toward CER and engagement in research from primary care clinicians.
PCORI Patient Survey	PCORI commissioned nationally representative surveys to collect information on attitudes toward CER and engagement in research from chronic disease and rare disease patients.
PCORI Caregiver Survey	PCORI commissioned nationally representative surveys to collect information on attitudes toward CER and engagement in research from caregivers of chronic disease and rare disease patients.
PCORI Researcher Survey	PCORI commissioned surveys to collect information about CER research practices, determinants of funding application submission, barriers to and facilitators of engaging patients and other stakeholders, and perceptions of PCORI programs.
Health Information National Trends Survey (HINTS)	HINTS collects nationally representative data routinely about the American public's use of cancer-related information. PCORI added survey items to assess awareness of, perceptions of, and interest in, engagement in health research. -
Stakeholder data collection	PCORI is commissioning efforts to collect opinions of stakeholder communities (initially, payers, clinicians, purchasers and policymakers) about PCORI's progress, to solicit input on future directions for PCORI via focus groups and interviews regarding PCORI's progress. Methods include focus groups, targeted interviews, and other targeted data collection efforts.

Stay tuned…

Questions?

Group Photo
followed by a
1 Hour Lunch Break
Lunch is served in Studio F.
We reconvene at 1:30 pm in this room.

Overview of Large Pragmatic Comparative Effectiveness Studies

Stanley Ip, MD

Senior Program Officer, Clinical Effectiveness Research

David Hickam, MD, MPH

Program Director, Clinical Effectiveness Research

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Key Questions for this Presentation

- What are the important features of patient centered outcomes research (PCOR)?

- What is the purpose of PCORI's pragmatic studies initiative?

- How does the Pragmatic Studies PFA differ from other PFAs?

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What Types of Research Does PCORI Support?

From the Authorizing Legislation:

"The terms 'comparative clinical effectiveness research' and 'research' mean research evaluating and comparing health outcomes and the clinical effectiveness, risks, and benefits of 2 or more medical treatments, services, and items…"

What is Evidence-based Information?

- Clinical evidence: Valid data about the outcomes experienced by patients who receive medical care.
  - The population is well defined.
  - The clinical interventions are well defined.
  - We have information about the most important outcomes (both benefits and harms).
- Comparative effectiveness
  - Focus on the choices people make about the options for managing a disease.
  - Compare the benefits and harms associated with each option.

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Shared Perspectives on Comparative Effectiveness Research

- Comparative Effectiveness Research should be a public good that:

  - Gives health care decision makers – patients, clinicians, purchasers and policy makers – access to the latest open and unbiased evidence-based information about treatment options

  - Informs choices and is closely aligned with the sequence of decisions patients and clinicians face

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What are the Features of Patient Centered Outcomes Research?

Patient-Centered Outcomes Research (PCOR) is the model for conducting research that addresses comparative effectiveness questions.

PCOR has the following characteristics:

- Actively engages patients and key stakeholders throughout the research process.

- Compares important clinical management options.

- Evaluates the outcomes that are most important to patients.

- Addresses implementation of the research findings in clinical care environments.

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What is a Pragmatic CER Study?

- Answers a practical, real world comparative effectiveness research question that is important to patients and decision makers

- Assesses whether two or more options differ in effectiveness when administered as they are in real life, and is conducted in a clinical setting that is as close as possible to a real world setting

- The methodological approach (including study design, outcome measures, and follow-up) is as simple as possible without sacrificing scientific rigor

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Objective of the Large Pragmatic Studies Program

- Generate evidence to provide useful information concerning which approaches to care might work best, given particular concerns, biology, settings, and preferences of the individuals
  - By necessity, these studies must be sufficiently large to allow rigorous comparisons of subgroups of interest

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ALLHAT, example of a pragmatic trial

- Antihypertensive trial
- Thiazide type diuretic vs. calcium channel blocker vs. ACE inhibitor
- >33,000 participants; 55 y/o+; HTN; 1 other risk factor
- Diverse representation with adequate subgroups of interest (e.g., African Americans, patients with diabetes)
- Follow up 4 to 8 years; study outcomes assessed at follow up visits; hospitalized outcomes based on clinic investigator reports

Traditional randomized controlled trials

- Study sample tends to be homogeneous, highly motivated (and therefore more adherent), relatively free of comorbid conditions

- Research tends to take place in specialized research settings

- Research protocols are often strict and not representative of typical clinical practice

Common practice in a Large Pragmatic Study

- Loose eligibility criteria
- Flexibility in application of the intervention of interest
- Outcomes assessed in usual circumstances
- Few or no follow up specifically for research purposes
- No special strategy for adherence

Pragmatic studies PFAs vs. other PFAs

- Expect to have major impacts on patients, healthcare practices, and directions of future research
- Involvement of major stakeholder/patient organizations as research partners is mandatory
- Target specific priority topics
- More resources ($10 million vs. $5 million or less)
- 5 years vs. 3 years or shorter

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Engage stakeholders/patients to help

- Formulate research questions
- Design the study to
  - integrate with routine clinic/office operations
  - minimize disruption to participant's daily routine
- Refine recruitment strategies and proactively deal with recruitment issues
- Participate in data monitoring and safety activities
- Capitalize on existing resources (e.g., electronic health records, claims databases, networks)
  - to collect study outcomes information
- Disseminate the study findings

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PCORI Priority Topics (as of 10/2014)

- Diagnosis and management of <u>bipolar disorder in children and adolescents</u>
- Management of breast <u>ductal carcinoma in situ</u> (DCIS)
- <u>Reduction of cardiovascular disease</u> (CVD) risk in underserved populations
- <u>Preventing the progression of episodic acute back pain</u> into chronic back pain
- <u>Integration of mental and behavioral health services</u> into the primary care of the <u>general population</u>
- <u>Integration of mental and behavioral health services</u> into the primary care of <u>persons at risk for disparities</u> in health care and outcomes
- Effectiveness of innovative strategies for enhancing <u>patients' adherence to medication regimens</u>.
- Treatment strategies for adult patients with <u>migraine headache</u>
- Medical vs. invasive procedures for <u>asymptomatic carotid artery stenosis</u>
- Surgical options for <u>hip fracture</u> in the elderly
- <u>Pelvic floor mesh</u>

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PCORI Priority Topics

- Effectiveness of specific features of <u>health insurance</u> on access to care, use of care, and other outcomes that are especially important to patients.
- Treatment strategies for symptomatic <u>osteoarthritis</u> (OA), including joint replacement
- Treatment strategies for patients with <u>autism spectrum disorder</u>
- Strategies for follow-up of <u>pulmonary nodules</u> identified by imaging studies
- <u>Proton beam therapy</u> for patients with lung, breast, and prostate cancer
- Biologic agents in the management of patients with <u>Crohn's disease</u>
- Active involvement by patients and caregivers in the management of <u>chronic</u> <u>mental illness</u>
- Multi-component interventions to reduce initiation or promote cessation of <u>tobacco use</u> among high-risk populations with known disparities
- Benefits and harms of continuous ambulatory peritoneal <u>dialysis</u> compared with hemodialysis
- Treatment options for people with <u>opioid substance abuse</u>
- Treatment options for patients with <u>multiple sclerosis</u>

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Questions and Answers

Thank you!

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Update on Pipeline to Proposals Awards

Courtney Clyatt, MPH
Senior Program Associate on Patient Engagement

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Pipeline to Proposal Initiative Update

- **Changes to the Pipeline to Proposal Initiative**
  - Working with Colorado Foundation for Public Health and the Environment to refine and streamline the Pipeline to Proposal Initiative. We have refined RFP, review, and awarding process and will now only be accepting new applicants for Tier I and Tier III.
  - Revised criteria for moving on to Tier II
  - Awardees will now have 21 months (9 months for Tier I and 12 months for Tier II) to build partnerships, develop their projects, and determine their CER question, after which they will have an opportunity to apply to a Tier III.
- **Awardee Management**
  - Three regional Pipeline Award Program Offices (PAPOs) have been selected for the Midwest, South, and East Regions, as well as a National Office has bas we are expanding the program across the nation.

| Tier I | Tier II | Tier III | PCORI Funding Announcement |

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Highlights from P2P Midpoint Reports

Overall, projects are moving forward and have met their deliverables. Awardees are reaching out to their regional, national, and, in some cases, international advocacy organizations.

- Parkinson's disease project has a communications partnership with **Parkinson's Association of the Rockies. PAR** now features information about and for the Wyoming Parkinson's disease support groups on its website.
- Lung cancer project has developed a partnership with the **American Lung Association. ALA** expressed an interest in partnering with this project on PCOR on both a state and national level.
- Alzheimer's disease project has formed a collaborative partnership with the national **Alzheimer's Association** and Montana's **Alzheimer's Association** chapter**.**
- A partnership was established with the **UK Sepsis Trust**.
- Project Lead for miners project was invited to speak at the **National Black Lung Coalition Annual Conference.**
- Project Lead for lung cancer project was invited to speak at **American College of Surgeons Clinical Congress** this October in San Francisco.
- Project Lead for a childhood obesity project was invited to testify before the **New Mexico Legislative Health and Human Services Committee.**
- Project on pre-term birth is now collaborating with the **Colorado Department for Public Health and the Environment** and **Denver Public Health** to improve maternal-child health in Colorado, using state data for project.

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Highlights from P2P Midpoint Reports

Awardees were asked to describe any accomplishments or benefits derived from their partnership that were not anticipated when they planned the project.

- **Sepsis Alliance** – As a result of networking through the Sepsis Alliance, an international partnership has developed with the UK Sepsis Trust, who assisted in the development of a survey about post-sepsis syndrome. The NIH ProMIS tools were adapted into our survey design to characterize the post-sepsis syndrome. The collaboration with the UK Sepsis Trust has allowed survivors in California to feel further validated by meeting additional physicians and survivors who were suffering from the same after-effects of sepsis, but live in a different country.

  In April 2014, Kaiser Permanente invited the Sepsis Alliance and its patient partners to speak about their personal experiences with sepsis. This Kaiser system-wide Sepsis Forum was attended by several hundred clinicians and nurses.

  In their midpoint report, the Project Lead stated that the research team has learned a great deal about sepsis from the patients themselves, rather than from traditional research laboratories.

- **Cystic Life** – The Project Lead has been pleasantly surprised by the amount of community interest. He has received many positive responses to their newsletter announcing the project and numerous applications to be part of their first-ever research advisory board, comprised of patients, physicians, researchers, parents, and others who serve in various capacities in the cystic fibrosis community. They have created a new program that was not part of their original plan because of the overwhelming interest in this project: CysticLife Research Ambassadors.

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Highlights from P2P Midpoint Reports

Awardees were asked to describe any accomplishments or benefits derived from their partnership that were not anticipated when they planned the project.

- **Sol Survivors** – The Project Lead noted that the greatest outcome of receiving this award has not been the funds, but the doors that have been opened by being able to say: "Hi, I'm a melanoma survivor, and I am also the director of a pilot project funded by the Patient-Centered Outcomes Research Institute." She's found that, in an academic setting, faculty members and other researchers are driven by grants, publications, and promotion and tenure. Because of this culture, having an award from PCORI has led researchers to treat her with a different level of respect than they have in the past.

- **Billings Health Clinic** – The Project Lead noted that they are working with patients, caregivers, and others in the Alzheimer's community to develop a Montana Alzheimer's State Plan, which was not a goal in their original proposal. They anticipate that creating a state plan for Montana should help them to identify a research collaboration idea for a Tier II award. This state plan also has the potential to benefit Montanans in other ways. It has the potential to increase public awareness about this major public health crisis and elevate Alzheimer's disease and other dementias as a priority for patient advocacy agencies, healthcare organizations and providers, state policy makers and healthcare agencies, and Montana communities. Montana will be designated a dementia-capable state as defined by Alzheimer's Association guidelines.

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Visit to Stomach Cancer Summit for P2P Project "Making Stomach Cancer a Priority for Asian Americans"

- About the Summit:
  - The purpose of the summit was to give the background on stomach cancer in the US, the world, and the Asian community.
  - Pre- and post-surveys were conducted to assess the impact of the summit on attendee's knowledge and attitudes toward stomach cancer.
  - There were 93 people in attendance, including clinicians, patients, community stakeholders, a Washington State Rep, and representation from the Washington State Commission on Asian Pacific American Affairs, as well as representatives from the Washington State Department of Health, and local insurance company representatives.

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Stomach Cancer Summit Highlights
Why Focus on Stomach Cancer?

- Stomach cancer is the second leading cause of cancer death worldwide, but it is a rare cancer in the US.

Legend:
- <5.9
- <9.6
- <14.6
- <22.0
- <70.0

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Stomach Cancer Summit Highlights
Stakeholder Input

- Scientific data on stomach cancer disparities

- Patients and volunteers at Cornerstone identified this as an issue affecting their community

- Korean American Health Professional Association Conference identified this as an issue affecting their community

- Fred Hutchinson Cancer Research Center's Korean American Community Advisory Board identified this as an issue affecting their community

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Stomach Cancer Summit Highlights
Risk Variations by Ethnicity

- There are significant ethnic variations in stomach cancer risk
  - Highest in Korean and Korean-American men
  - Higher for all minorities

Stomach Cancer Incidence by Race & Ethnicity

	Pacific Islanders	Asian Americans	African Americans	Hispanic Americans	American Indians/ Alaskan Natives	Caucausian Americans
Men	18.8	17.1	15.1	15.0	13.6	8.7
Women	14.5	10.6	8.8	8.1	7.3	3.7

■ Men ■ Women

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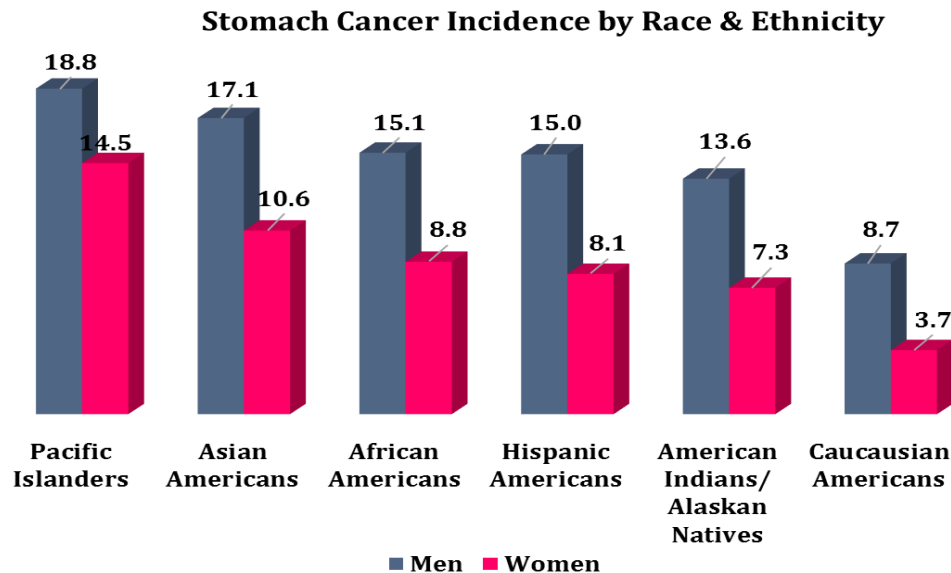
Promising Advancements in Stomach Cancer Screening and Treatment

- Screening programs in South Korea and Japan have increased detection and improved survival rate for stomach cancer
  - In Japan, the 5-year survival rate is 62.1
  - In Korea, the 5-year survival rate is 67

- In the US, where screening is not regularly practiced, the 5-year survival rate is 26.9

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Stomach Cancer Summit Highlights
Goals

- ### Goal of the Summit
  - Identify what is needed to address this topic and conduct more scientific research
  - Determine how stakeholders can work together as partners to develop an action plan (group discussions, Stomach Cancer Advisory Board)

- ### Overall Goal of the Project
  - Impact stomach cancer guidelines

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Stomach Cancer Summit Highlights
Project Partner

- Project Partner – Fred Hutchinson Cancer Research Center (FHCRC)
- Role of FHCRC
  - To partner with the community to address issues of concern
  - FHCRC serves as a scientific advisor
  - Provide guidance on:
    - Evidence-based practices, research design, grant writing, training
  - Advance the science around stomach cancer and increase awareness of this issue in the scientific community
- FHCRC hopes to impact stomach cancer screening guidelines for high-risk populations

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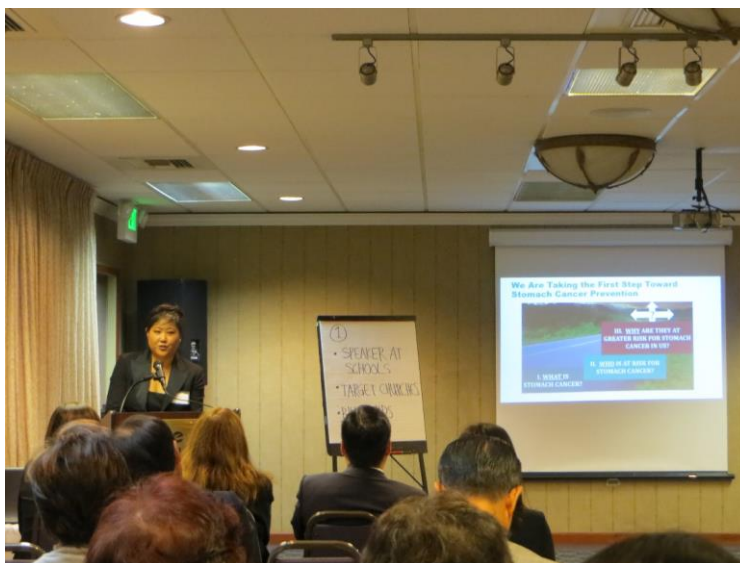
Project Staff, Interns, and the Federal Way Deputy Mayor

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15 Minute Break

Refreshments outside.

Update on the PCORI Ambassador Program

Sue Sheridan, MIM, MBA
Director of Patient Engagement

Suzanne Schrandt, JD
Deputy Director, Patient Engagement

Aingyea Kellom, MPA
Program Associate, Patient Engagement

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Objectives

- Program Update

- Program Evaluation – Midpoint Survey Results

- Panel Discussion: Why Build a Networked Community?

- Breakout Session: Strategies for the Future

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Program Update (3:45 - 3:55 p.m.)

New Team Member: Rashonda Welch

- From Atlanta, Georgia
- National Urban Fellow, completing Mentorship with PCORI from September 2014-May 2015
- Project Focus: Development of a national, multi-stakeholder network for PCORI's Ambassador Program through social media and other innovative communications strategies
- 2015 MPA Candidate at Baruch College-CUNY

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PCORI's New Website Launched!!!!

- Each Ambassador has
profile page with interest areas

- Ambassadors listed by region

- Added video footage

- Ambassadors listed if….

  ✓ Completion of PCOR Science Training
  ✓ Provided Consent
  ✓ Bios
 ✓ Letter of Support (Organizational
    Ambassadors)

Revisions to Training Exam

Old Exam

- 24 questions

- Tested memory

- More than one answer could be arguably correct

- Test retakes would shuffle exam questions

- Missing useful content on the role of Ambassador
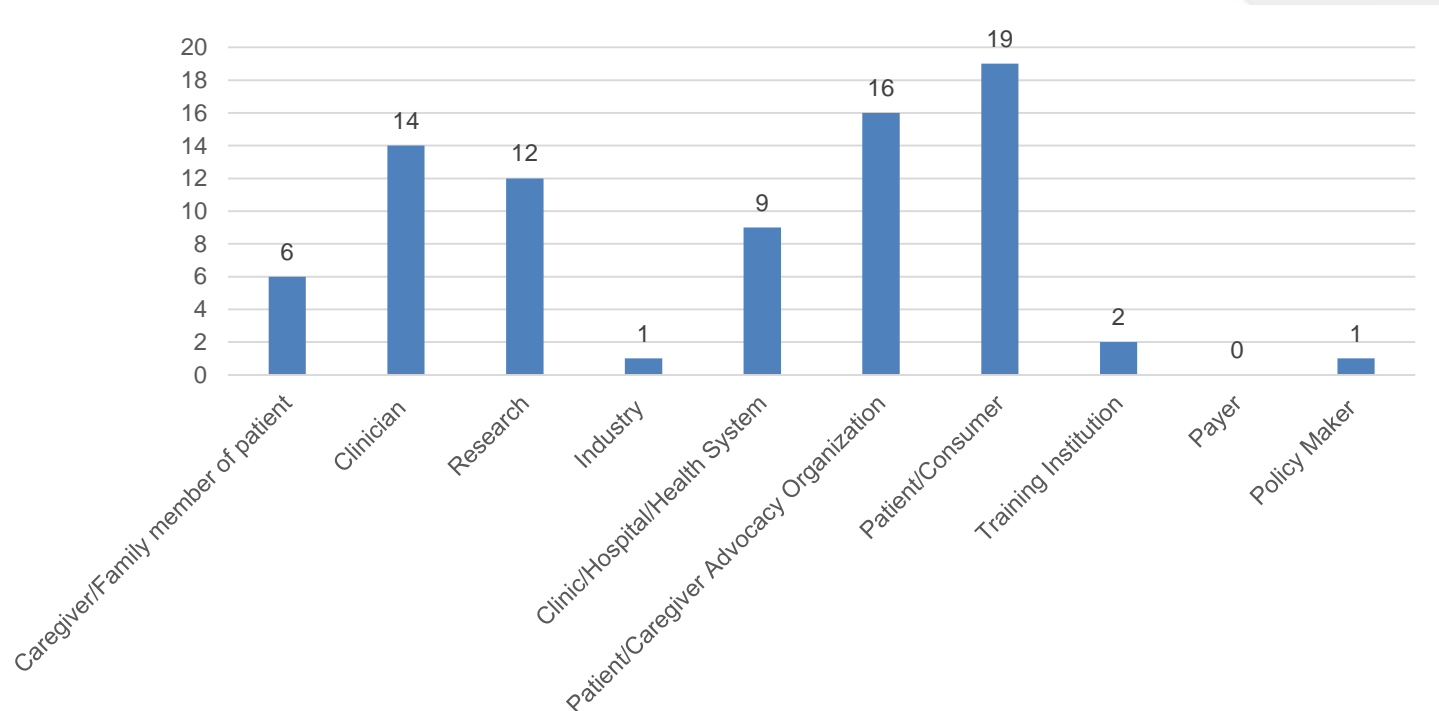
New Exam

- 23 questions

- Tests understanding

- Incorrect answers revised to be more wrong

- Exam questions no longer shuffled during retakes

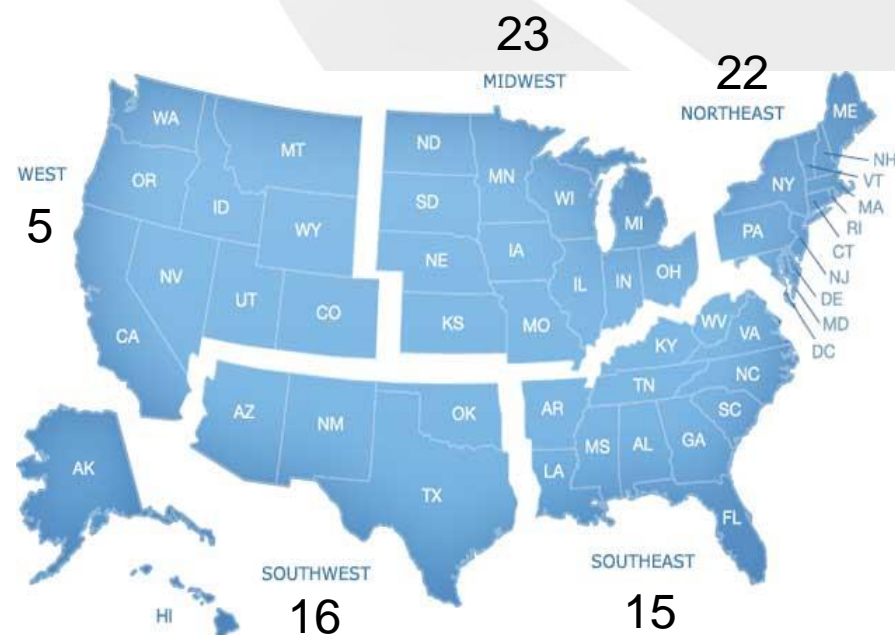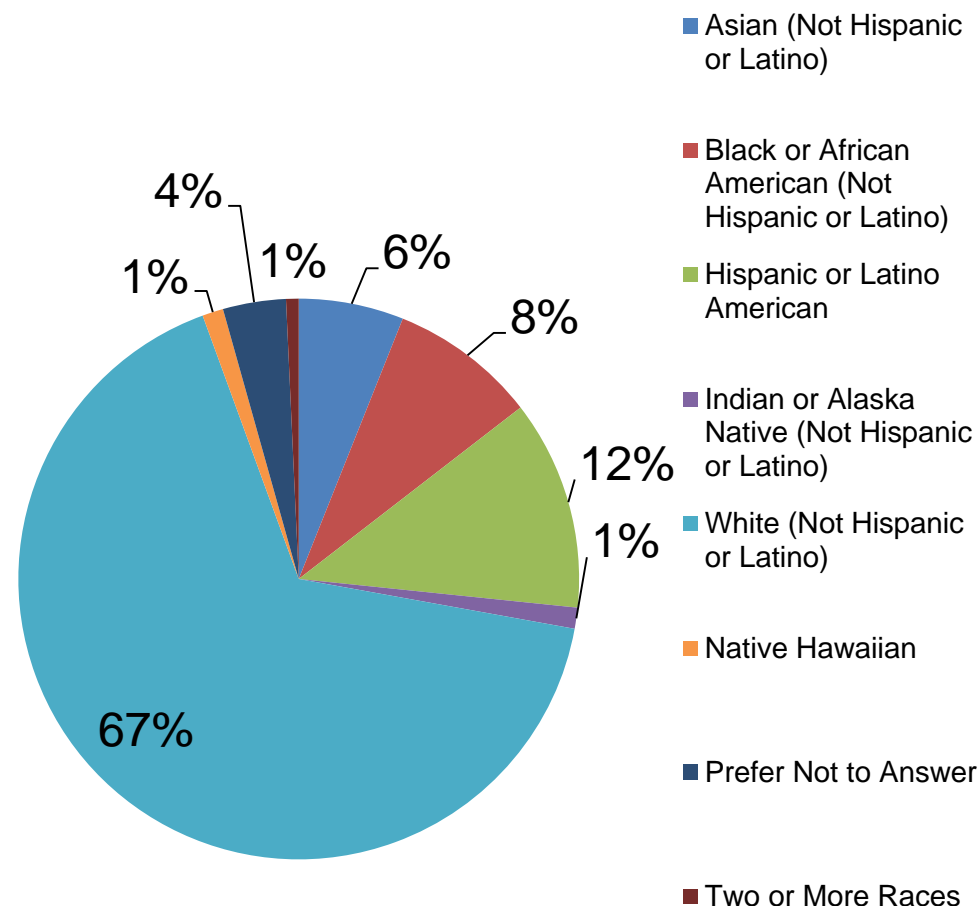- New content added

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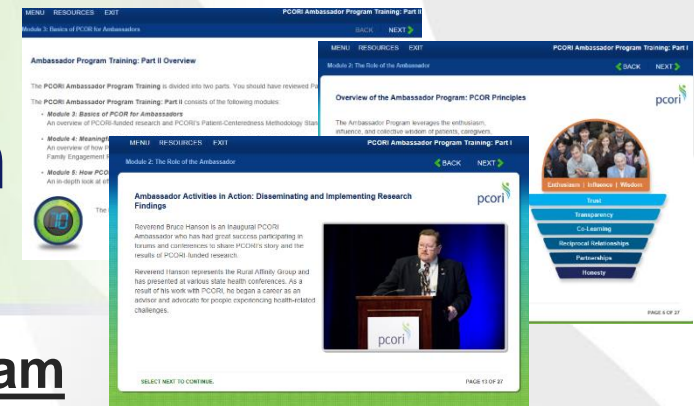
Ambassadors by Stakeholder Group

- To Date: 81 have completed the Ambassador Training
  - 65 individual and 16 organizational

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Ambassadors by Ethnicity and Region

Legend:
- Asian (Not Hispanic or Latino)
- Black or African American (Not Hispanic or Latino)
- Hispanic or Latino American
- Indian or Alaska Native (Not Hispanic or Latino)
- White (Not Hispanic or Latino)
- Native Hawaiian
- Prefer Not to Answer
- Two or More Races

Pie chart values: 4%, 1%, 1%, 6%, 8%, 12%, 1%, 67%

Map region values:
- WEST: 5
- MIDWEST: 23
- NORTHEAST: 22
- SOUTHWEST: 16
- SOUTHEAST: 15

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16 Organizational Ambassadors by State
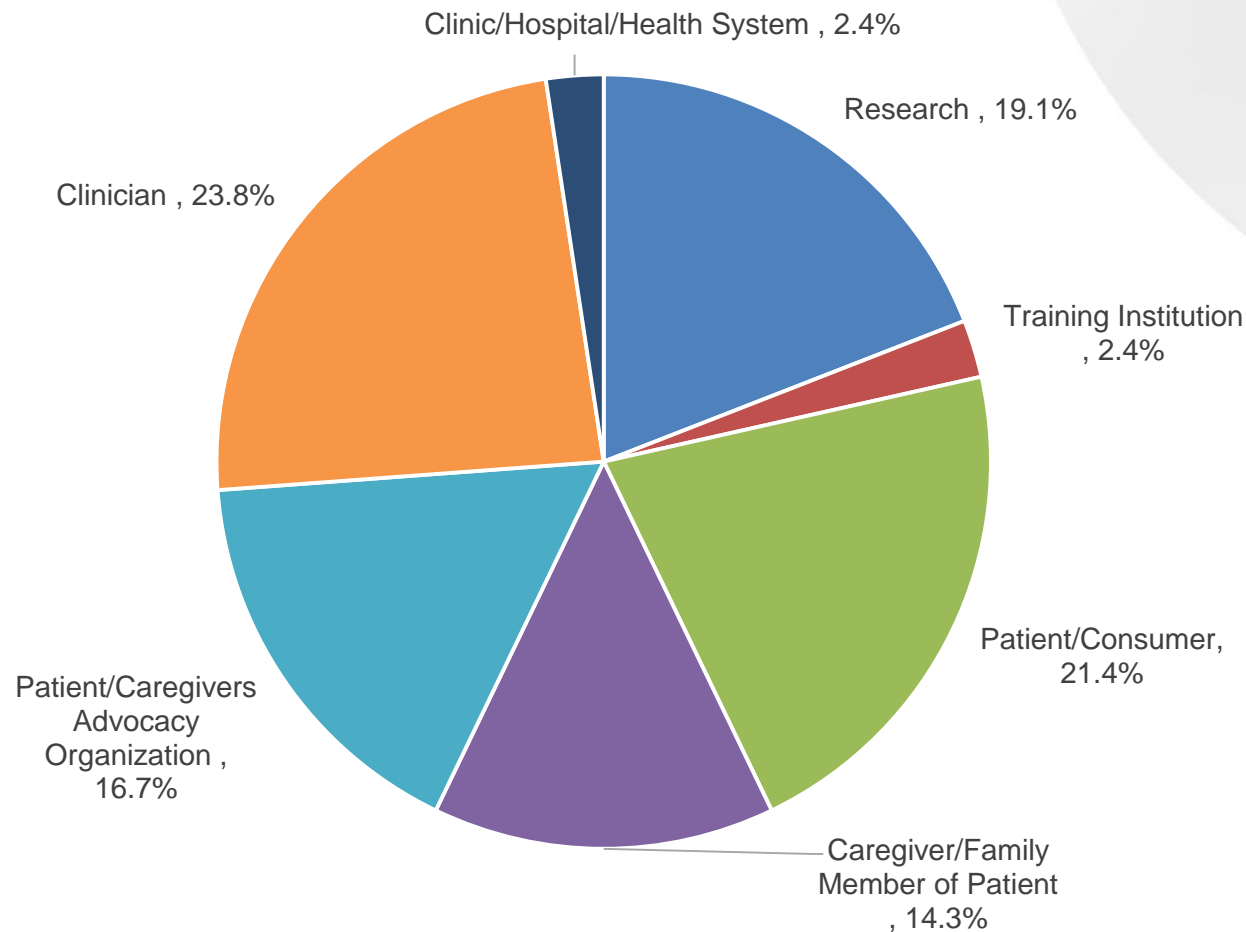
Organization	State
Patient-Centered Primary Care Collaborative	DC
Association of periOperative Registered Nurses	CO
Mercy Health Chicago	IL
The S.T.A. R. Initiative	IL
National Patient Advocate Foundation(NPAF), and the American Heart Association(AHA)	CA
univ of nm dept of psychiatry	NM
FasterCures	DC
Epilepsy Foundation Central & South Texas	TX
American Occupational Therapy Association	MD
The American Academy of Neurology	MN
Dia de La Mujer Latina	NY
International Cancer Advocacy Network (ICAN)	AZ
Association of Nurses in AIDS Care	PA
American College of Physicians	PA
National Association of Nurse Practitioners in Women's Health	WV
Dia de la Mujer Latina	TX

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Midpoint Survey Results (3:55 p.m. - 4:10 p.m.)

Survey Responders by Stakeholder Groups

Response Rate: 55%

Pie chart showing:
- Clinic/Hospital/Health System, 2.4%
- Research, 19.1%
- Training Institution, 2.4%
- Patient/Consumer, 21.4%
- Caregiver/Family Member of Patient, 14.3%
- Patient/Caregivers Advocacy Organization, 16.7%
- Clinician, 23.8%

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Participating in the program has increased my understanding and knowledge of PCOR.

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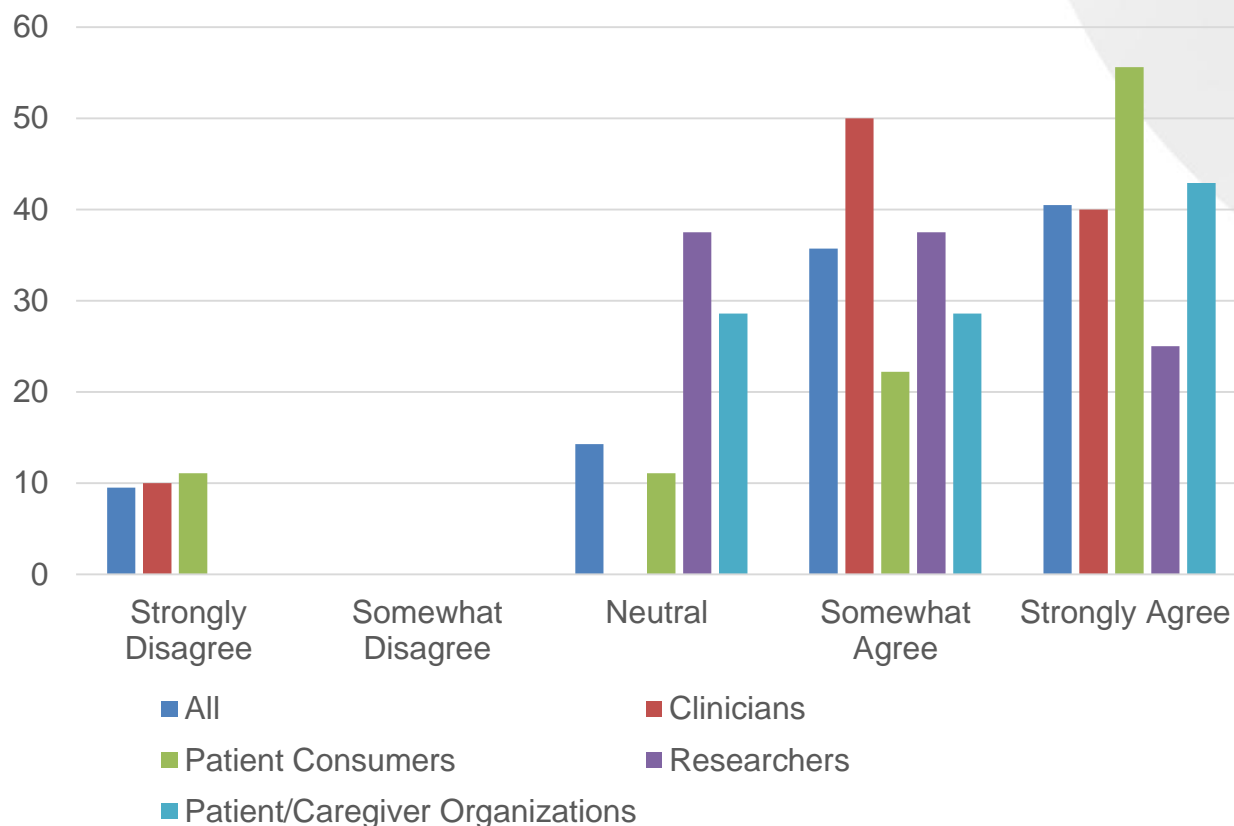
By participating in the PCORI Ambassador online community, I was able to build new relationships with other Ambassadors.
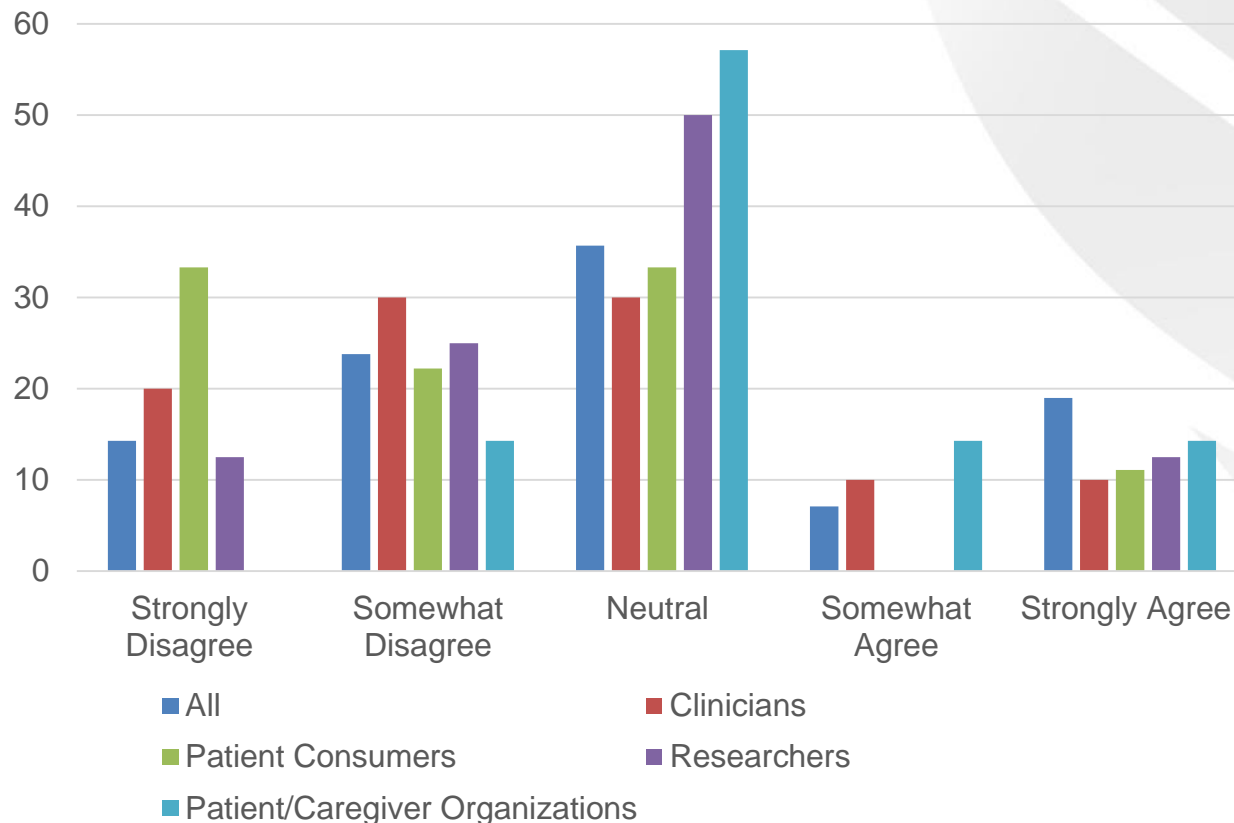
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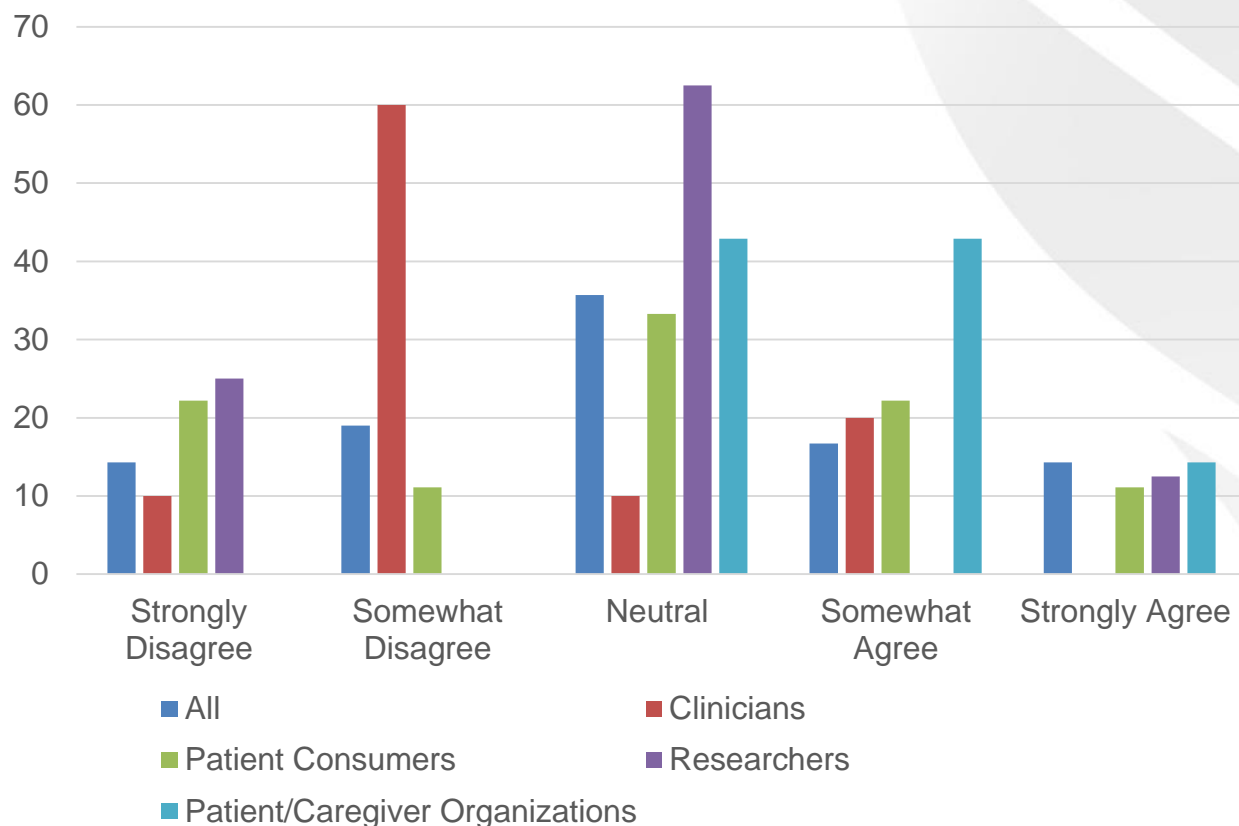
My participation in the program was worthwhile.

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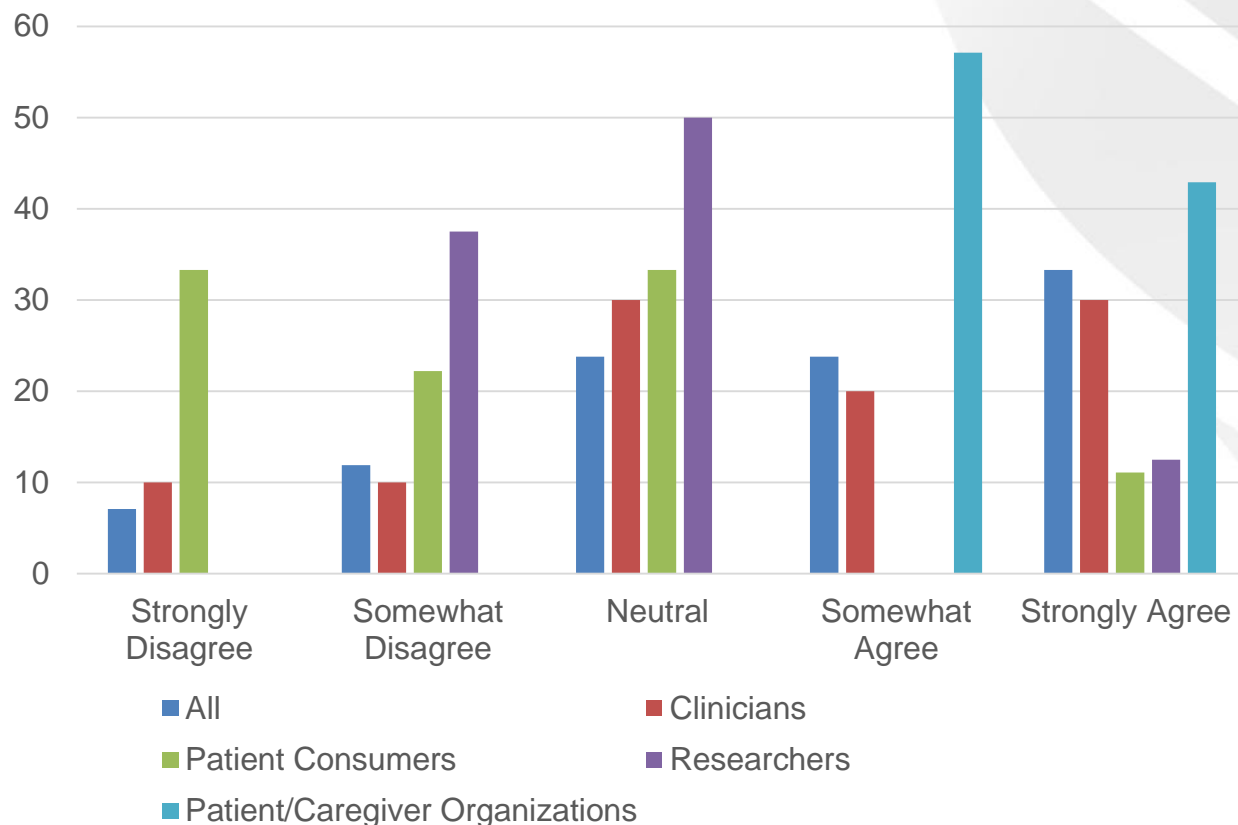
By participating in the PCORI Ambassador Program, I was able to build new research partnerships.

Legend:
- All
- Clinicians
- Patient Consumers
- Researchers
- Patient/Caregiver Organizations

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My participation in the program broadened my perspective on including patients and other stakeholders in research.

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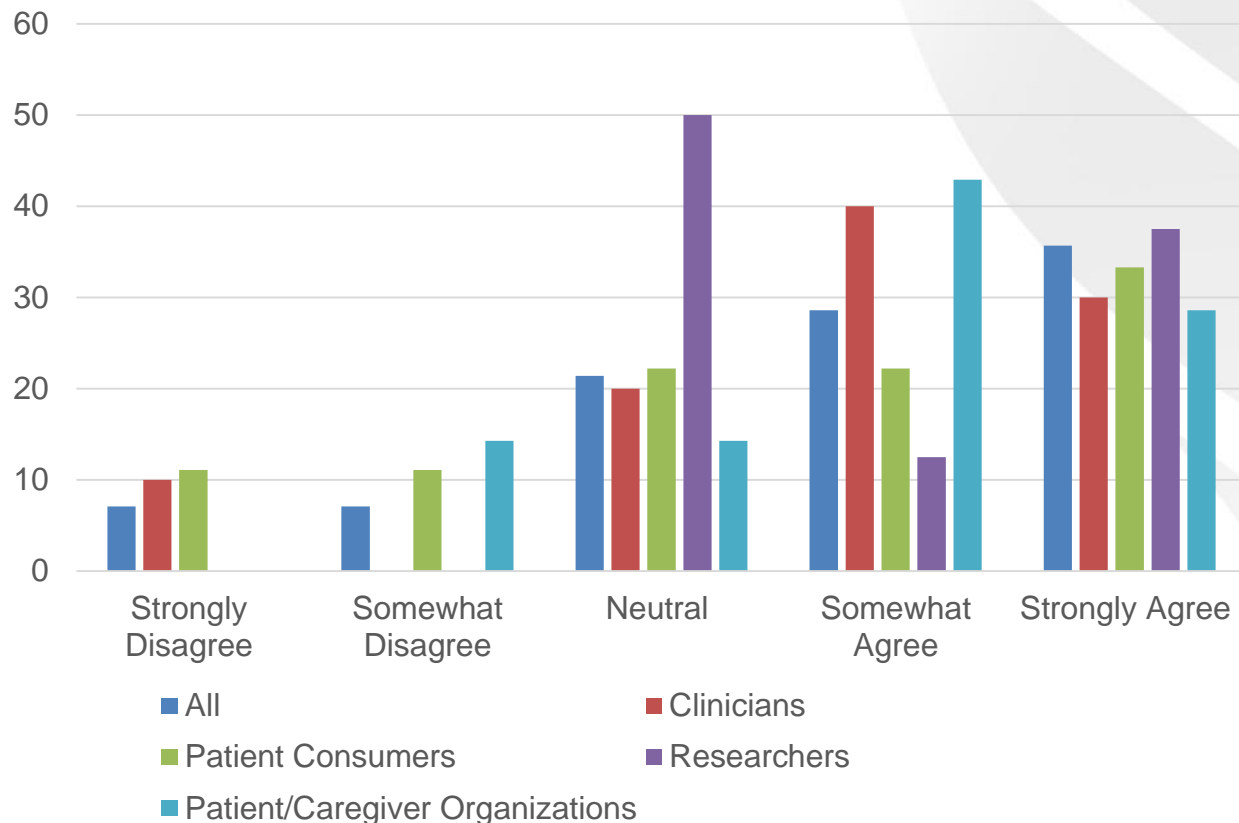
I have completed the following Ambassador Program activities:

Legend:
- Featured in a PCORI blog
- Submitted an op-ed to a periodical
- Conducted a presentation
- Submitted an application for an Advisory Panel
- Submitted an application for a Funding Announcement
- Submitted an application for a Eugene Washington Award
- Other

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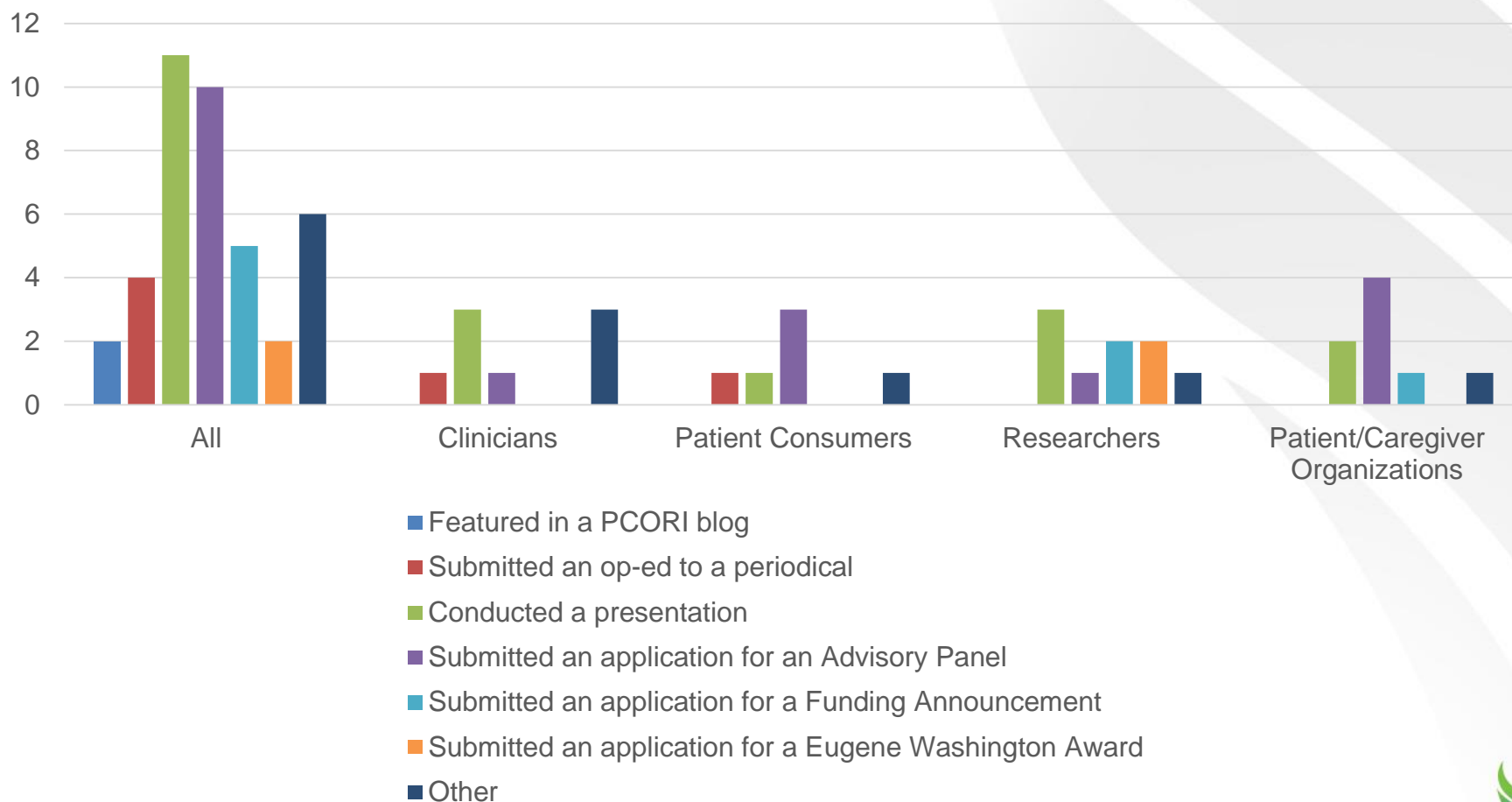
Next Steps

- Share information with Ambassadors
  - Newsletter

- Use information for strategic planning

- Continue evaluation plan
  - Targeted interviews
  - Training course survey
  - Ambassador activity tracker (delayed)

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Panel Discussion (4:10 p.m. - 4:50 p.m.)

Why Build a Networked Community?

Panelists:

- Lily Cappelletti
  - Associate Director, Research Partnerships
  - The Michael J. Fox Foundation for Parkinson's

- Anindita (Annie) Saha
  - Director, External Expertise and Partnerships
  - FDA/CDRH/OCD

- Jamie Sullivan
  - Director, Public Policy
  - COPD Foundation

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Breakout Session (4:50 p.m. - 5:15 p.m.)

Strategies for the Future

- Areas for Growth: As we contemplate opening the program up to the public, how should we build upon our participation? Possibilities to consider include:
  - Aligning growth with scientific priorities, such as the pragmatic clinical trials.
  - Working toward greater participation by our funded project teams.
  - Other individuals or organizations of particular interest.
- Value Added: What benefits can the Ambassador program provide to participants? What would make involvement the most valuable to patients, researchers, and other stakeholders?
- Opportunities for Cross-fertilization: What are the best ways to collaborate with and learn from work going on across the healthcare system spectrum, such as that done by PFACs, IHI, FDA, NQF, CMS, AHRQ, and others?

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Questions?

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Wrap-Up

Sue Sheridan, MIM, MBA
Director of Patient Engagement

Patient-Centered Outcomes Research Institute

30 Minute Break

Dinner will begin at 6:00pm in Studio F.
Breakfast will begin at 8:00am tomorrow in Studio F.