



Involving patients in research: what have we done and how did we do it? Lessons learned from the Patient-Centered Outcomes Research Institute (PCORI).

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Patient-Centered Outcomes Research Institute

Overview

- **Overview of PCORI and patient-centeredness**
- **Patient engagement in research:**
 - The Patient and Family Engagement Rubric
- **Patient engagement in building a data research network:**
 - PCORnet
- **Building the evidence for patient engagement in research:**
 - Model for evaluating engagement in research
 - WE-ENACT (Ways of Engaging - ENgagement ACTivity Tool).and Net-ENACT (PCORnet ENgagement ACTivity Tool)

Engagement!



Why PCORI?



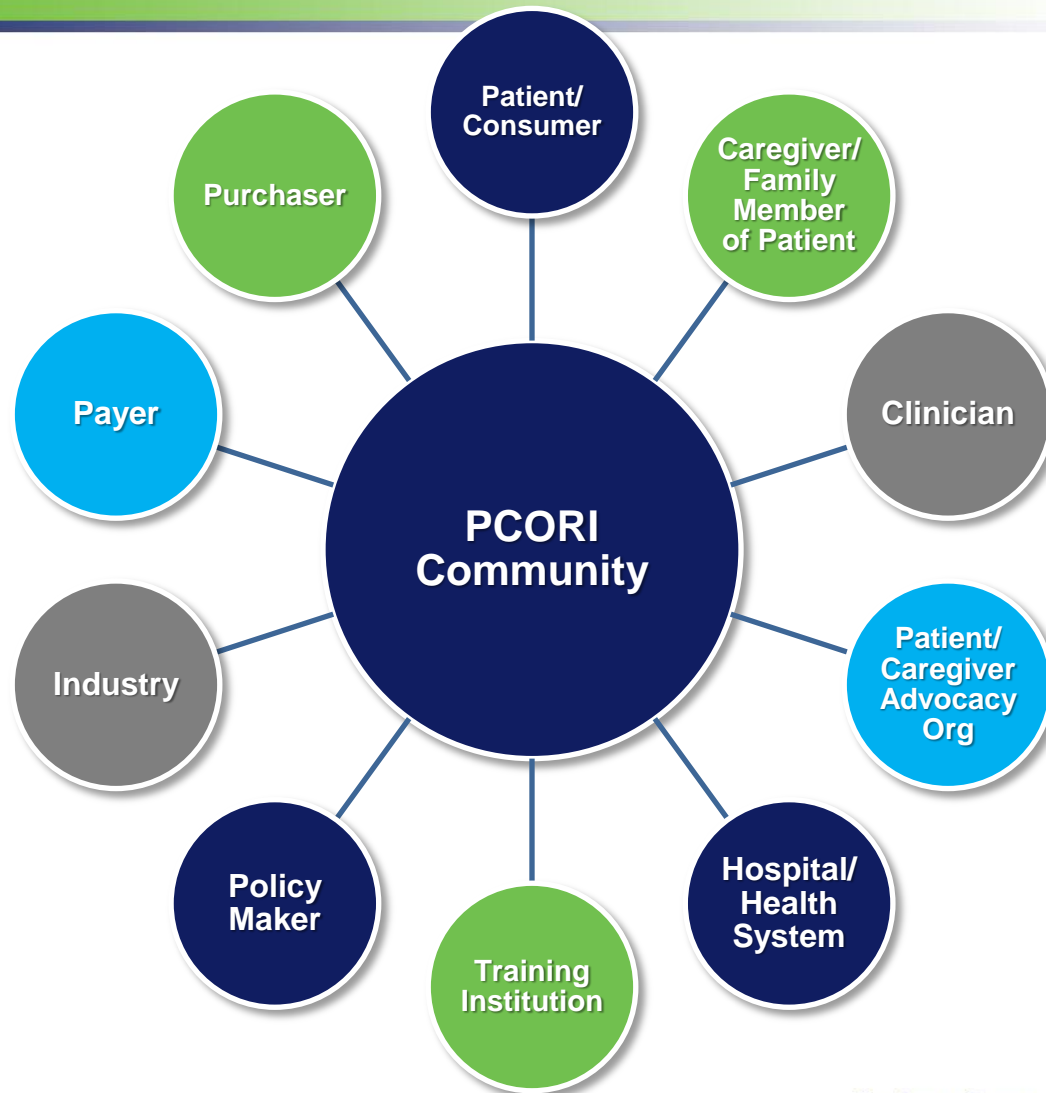
- Research has not answered many questions patients face
- People want to know which treatment is right for them
- Patients need information they can understand and use

PCORI's Mission

PCORI helps people make informed health care decisions, and improves health care delivery and outcomes, by producing and promoting high integrity, evidence-based information that comes from **research guided by patients, caregivers and the broader health care community.**



Who Are PCORI's Stakeholders?



PCORI Engages Patients and Other Stakeholders at Every Step



Why Engage Patients in Research?

Patient engagement in the design and conduct of research offers a greater likelihood of:

- influencing research to be patient centered, useful, and relevant
- establishing trust and a sense of legitimacy in its findings
- successful use and uptake of research results by the patient community.

Why Engage Patients in Research?

Patients contribute:

- 🌐 “Lived experience” of condition or disease
- 🌐 Research questions that are important to patients
- 🌐 Outcomes important to patients such as quality of life
- 🌐 Real world input on risk tolerance of interventions
- 🌐 Power of data (new currency)
- 🌐 Focus on patient-centeredness, ethics, safety, and urgency
- 🌐 Connections to other patients like them, which facilitates recruitment and dissemination of research findings

Patient-Centeredness vs. Patient Engagement

- **Patient- Centeredness** is a component of what PCOR is looking for in research applications
 - Does the project aim to answer questions or examine outcomes that matter to patients within the context of patient preferences?
 - Research questions and outcomes should reflect what is important to patients and caregivers
- **Patient engagement** is about having patients as partners in research as opposed to merely subjects
 - Active engagement between scientists, patients, and stakeholders
 - Community, patient, and caregiver involvement already in existence or a well-thought out plan



What does patient engagement look like in the planning, conduct and dissemination of research?

The Patient and Family Engagement Rubric

Why develop a rubric?

- The rubric is a response to frequent questions from the patient and research communities asking what we mean by “engagement in research.”

What is the rubric?

- The rubric is a framework that provides a variety of options for incorporating engagement, where relevant, into the research process.

How will the rubric be used?

- The rubric will be used as a guide for applicants, merit reviewers, awardees and Engagement Officers.

Rubric: Patient and Family Engagement in Patient-Centered Outcomes Research (PCOR)



Planning the Study



Conducting the Study



Disseminating the Study Results



PCOR Engagement Principles

Rubric: Planning the Study

Planning the Study
Formulating Research
Questions and Study

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.**

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Patient Engagement in Developing the Research Question



Elements of the Rubric



Planning the Study



Conducting the Study



Disseminating the Study Results



PCOR Engagement Principles

Rubric: Conducting the Study

Conducting the Study

Participating in and monitoring the conduct of the project

Patient and research

Participating in and monitoring the conduct of the project

Patient partners participate in the recruitment of and data collection from the study participants, when appropriate.

Patient recruitment

The research team

partners to make it understandable to survey tool be adapted for children to “emotional well-being,” for the term, of the patient partners in conducting and component of the study, (e.g., helping to or readability, etc.), including the

recruit study participants and to their patient networks—the “book club” of the patient partners in interacting with ng with study participants (e.g.,

ruly involved in the research and if they

- include in your application a plan for “check-ins” with patient partners to monitor their perceptions of the extent to which a) they are meaningfully involved in the study and b) their participation is contributing to the study.
- Also include a plan for “check-ins” with the other research team members to monitor their perceptions of the extent to which a) patient partners are meaningfully involved in the study and b) their participation is contributing to the study.

Elements of the Rubric



Planning the Study



Conducting the Study



Disseminating the Study Results



PCOR Engagement Principles

Rubric: Disseminating the Study Results

Disseminating t

Helping to plan the dissemination of the study results.

Helping to plan the dissemination of the study's results.

Patient partners are involved in plans for disseminating the study's findings to patient, stakeholder, and research audiences so that the findings are communicated in understandable, usable ways.

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of patient partners in findings.

Rubric: Disseminating the Study Results

Dissemination

Helping to plan the dissemination of study results.

Examples

- **Chronic pain study: patient partners co-author manuscripts, present at scientific and lay conferences, and share study findings through their networks.**
- **Cardiac study: a Patient Dissemination Board is helping to craft the dissemination plan and advise the research team on how to best share study findings.**

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Elements of the Rubric



Planning the Study



Conducting the Study



Disseminating the Study Results



PCOR Engagement Principles

Rubric: PCOR Engagement Principles

PCOR Engagem
Reciprocal Relationsh

Reciprocal Relationships

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

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Rubric: PCOR Engagement Principles

PCOR Engage

Co-learning

Co-learning

The application includes plans to ensure that the patient partners will understand the research process and the researchers will understand patient centeredness and patient engagement.

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Rubric: PCOR Engagement Principles

<p>PCOR Engagemen</p>	<h2>Partnership</h2>	
<p>Partnership</p>	<p>Time and contributions of patient partners are valued and demonstrated in fair financial compensation, as well as reasonable and thoughtful time commitment requests.</p> <p>When the patient partners represent unique populations, the research team proposes to accommodate their cultural diversity and/or disability.</p>	<p>s included in the nts. ulation, several Hispanic and fluent in with a disability, the n meetings that are</p>

Rubric: PCOR Engagement Principles

PCOR Engage

Trust, Transparency
Honesty

Trust, Transparency, Honesty

- **Major decisions are made inclusively and information is shared readily with all research partners.**
- **Patient partners and research partners express commitment to open and honest communication with one another.**
- **The study team commits to communicate the study's findings back to the study community in a meaningful and usable way.**

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What does patient engagement look like in building a data research network?

PCORnet: the National Patient-Centered Clinical Research Network



The **goal** of PCORI's National Patient-Centered Clinical Research Network Program is to **improve the nation's capacity to conduct clinical research** more efficiently, **by creating a large, highly representative, national patient-centered clinical research network** with a focus on conducting CER – both randomized and observational.

The **vision** is to support a learning US healthcare system, which would allow for **large-scale research** to be conducted with **enhanced accuracy and efficiency** within real-world care delivery systems.



11 Clinical Data Research Networks and 18 Patient Powered Research Networks



Patient and Family Engagement in Data Network Development (PCORnet)

Enrollment and diversity

- Increasing size of the network
- Increasing the diversity of the network
- Retention of network members

Governance

- The development of the network governance structure, roles and responsibilities
- Development of procedures, bylaws and policies for the network

Data collection

- The development of data collection tools
- Identification of Patient Reported Outcomes (PROs) for inclusion in database

Data sharing, privacy and consent

- The development of consent processes and policies
- Development of data sharing agreements
- Development of privacy policies

Patients are Actively Shaping the Network

- Governance of the Network
- Cohort characterization
- Identifying important considerations for consent
- Determining plans for returning results to patients
- Identifying opportunities to inform patients about research in the care continuum

It's time for **patients** to have a voice in national healthcare conversations.



Join a Patient Engagement Panel

WE ARE OCHIN

You bring a valuable perspective to the national conversation surrounding healthcare delivery and research.

OCHIN is a nonprofit healthcare innovation center designed to provide knowledge solutions that promote quality, affordable healthcare to all.

www.ochin.org

Share that perspective and help shape future programs by joining OCHIN's Patient Engagement Panel, and contribute your thoughts on:

Details:

- Research questions
- Changes to healthcare processes and their impact on patients
- Project communication to the general public
- Patient recruitment for studies
- Health technology

- You will receive gift cards for time spent during PEP meetings.
- The Patient Engagement Panel meets monthly for one hour and focuses on primary care research and the patient perspective.

Interested?

Email or call Nate Warren, OCHIN Patient Engagement Coordinator, for more information about getting involved today: warren@ochin.org or 503.943.2568.

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Patient Powered Research Networks represent a number of conditions...

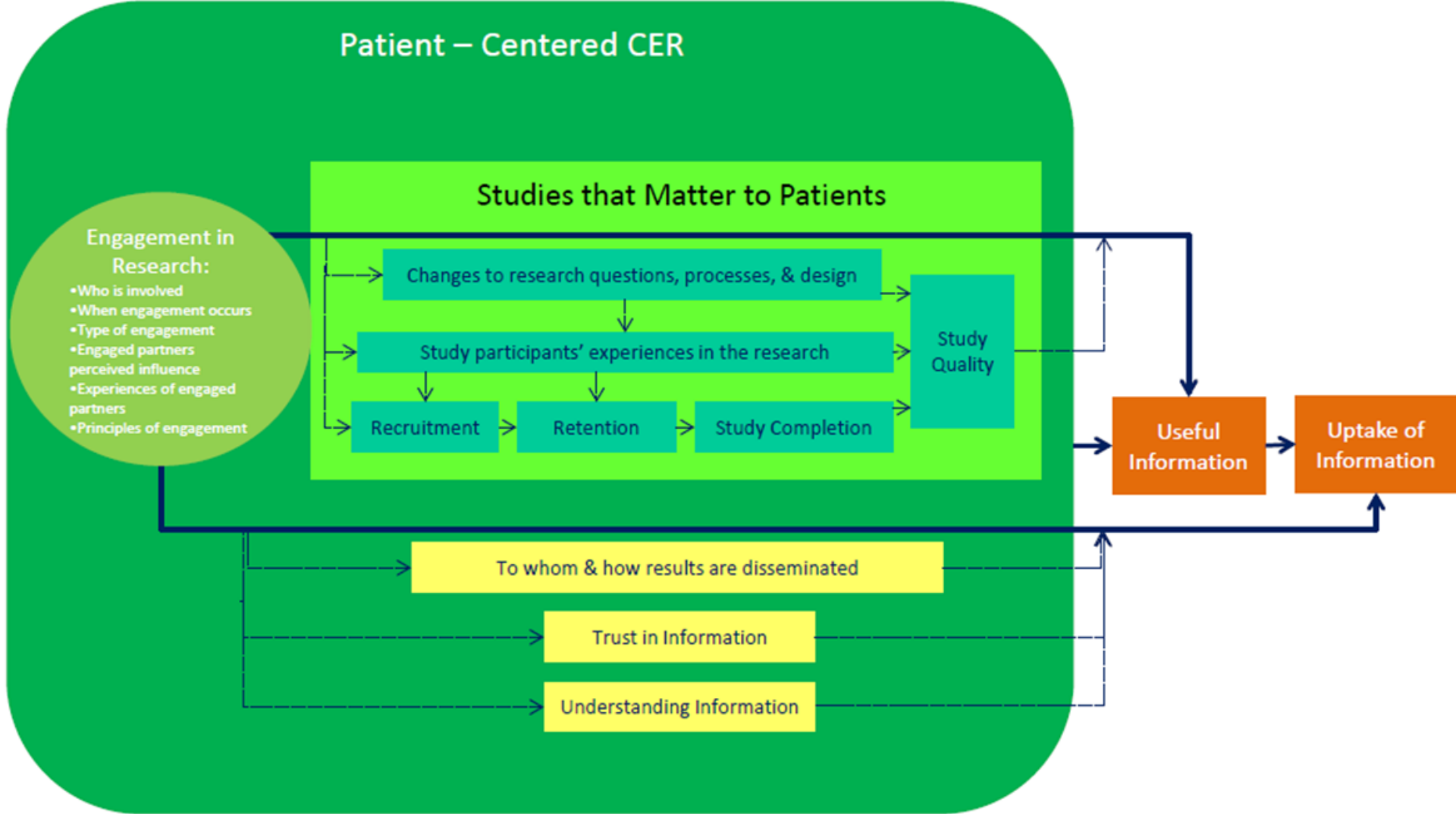
Organization	Condition	Pop
Accelerated Cure Project for Multiple Sclerosis	Multiple Sclerosis	20,000
American Sleep Apnea Association	Sleep Apnea	50,000
Cincinnati Children's Hospital Medical Center	Pediatric Crohn's Disease and Ulcerative Colitis	15,000
COPD Foundation	Chronic Obstructive Pulmonary Disease	50,000
Crohn's and Colitis Foundation of America	Inflammatory Bowel Disease (Crohn's disease and ulcerative colitis)	30,000
Global Healthy Living Foundation	Arthritis (rheumatoid arthritis, spondyloarthritis), musculoskeletal disorders (osteoporosis), and inflammatory conditions (psoriasis)	50,000
Massachusetts General Hospital	Major Depressive Disorder and Bipolar Disorder	50,000
University of California, San Francisco	Cardiovascular health	100,000
University of South Florida	Hereditary Breast & Ovarian Cancer	17,000

....including rare diseases

Organization	Condition	Population Size
ALD Connect, Inc	Adrenoleukodystrophy	3,000
Arbor Research Collaborative for Health	Primary Nephrotic Syndrome, Focal Segmental Glomerulosclerosis, Minimal Change Disease, and Membranous Nephropathy Multiple Sclerosis	1,250
Duke University	Juvenile Rheumatic Disease	9,000
Epilepsy Foundation	Aicardi Syndrome, Lennox-Gastaut Syndrome, Phelan-McDermid Syndrome, Hypothalamic Hamartoma, Dravet Syndrome, Tuberous Sclerosis	1,500
Genetic Alliance, Inc	Alström syndrome , Dyskeratosis congenital, Gaucher disease, Hepatitis, Inflammatory breast cancer, Joubert syndrome, Klinefelter syndrome & associated conditions, Psoriasis, Metachromatic leukodystrophy, Pseudoxanthoma elasticum,	50- 50,000
Immune Deficiency Foundation	Primary Immunodeficiency Diseases	1,250
Parent Project Muscular Dystrophy	Duchenne and Becker muscular dystrophy	4,000
Phelan-McDermid Syndrome Fndn	Phelan-McDermid Syndrome	737
University of Pennsylvania	Vasculitis	500

How do we build the evidence for patient engagement in research?

Model for Evaluating Engagement in Research



PCORI has three strategic goals

- Substantially increase the quantity, quality, and timeliness of useful, trustworthy information available to support health decisions
- Speed the implementation and use of patient-centered outcomes research evidence
- Speed the implementation and use of patient-centered outcomes research evidence

Increase Information

Merit Reviewer Survey Details

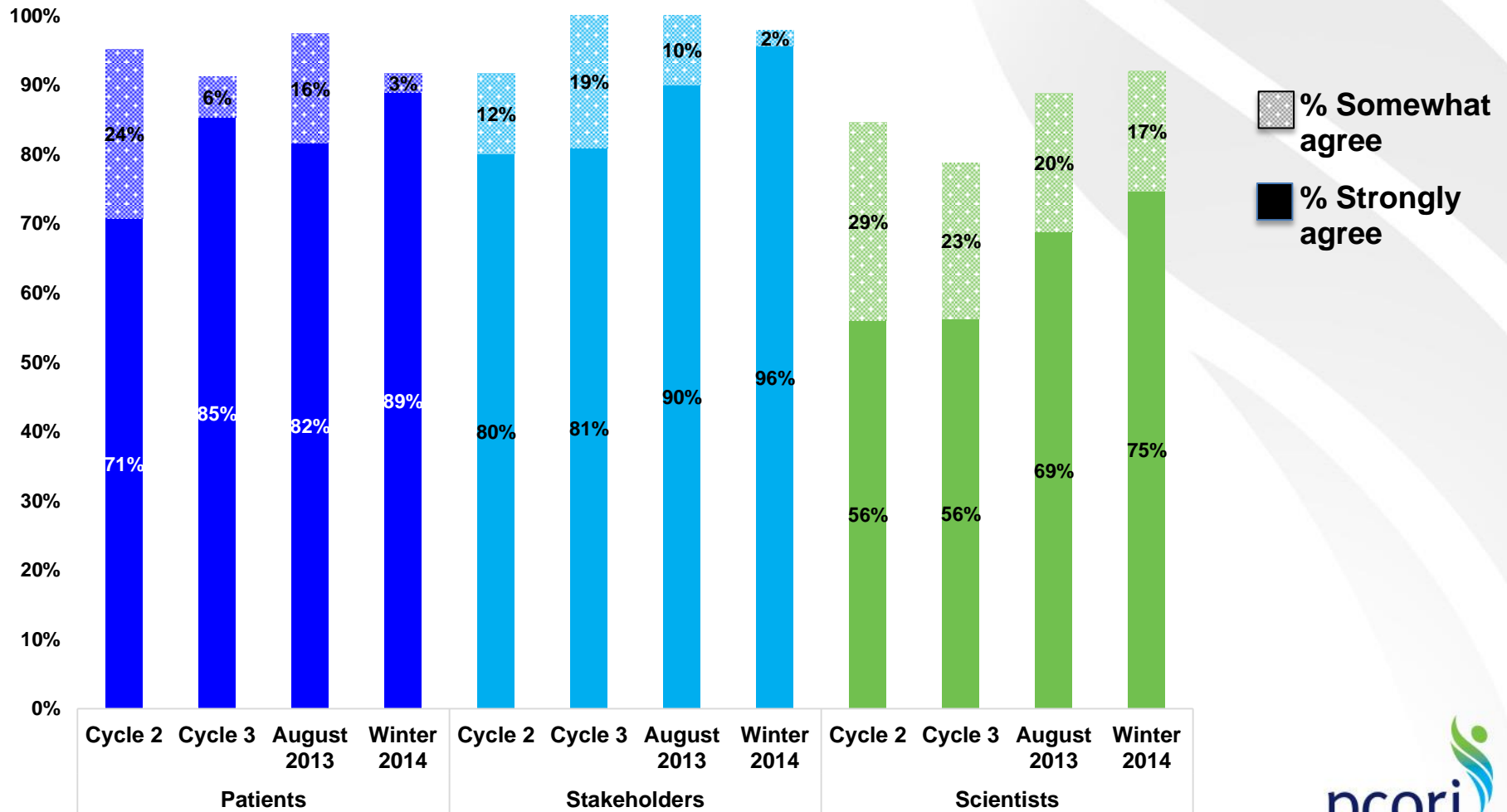
We asked merit reviewers to rate their agreement with the following statement:
The patient/stakeholder reviewers provided valuable input during the discussion

(Strongly Disagree, Somewhat Disagree, Neither Agree nor Disagree, Somewhat Agree, and Strongly Agree)

Survey	Sample Size	Survey Completion Rate	Percent Who Agree Somewhat or Strongly
Cycle II	205	94%	89%
Cycle III	167	97%	85%
August 2013	286	91%	93%
Winter 2014	209	83%	94%

Surveys of merit reviewers are conducted immediately following each in-person merit review and are live in the field for 2 weeks.

Percent of merit reviewers who agree with the statement: *“The patient/stakeholder reviewers provided valuable input during the discussion”*



Ways of Engaging- ENgagement ACTivity (WE-ENACT) Inventory

- Self-report tool for researchers, patients, and stakeholders
- Completed at baseline and on an annual basis
- Domains:
 - Who is engaged
 - Partnership characteristics
 - Level of engagement
 - When in research process are they engaged
 - Perceived level of influence of partners
 - Effects of engagement on research questions, study design, study implementation, and dissemination of results
 - Challenges, facilitators
 - Lessons learned for engagement
 - PCOR principles (respect, co-learning, etc.)

Find Us Online



www.pcori.org



Patient-Centered Outcomes Research Institute



Thank You

Jean Slutsky

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Program Director for Communication and Dissemination Research

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