

# PCORI ADVISORY PANEL ON PATIENT ENGAGEMENT

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*Summer 2019 Meeting*

Day 1

**June 27, 2019**

9am-4:45pm

# Public Policy Update

## (Closed Session)

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**Andrew Hu**

Director of Public Policy and Government Relations

**Caitlin McCormick**

Associate Director of Public Policy and Government  
Relations

# Webinar Housekeeping



- Webinar is available to the public and is being recorded
- Members of the public are invited to listen to this teleconference and view the webinar
- Anyone may submit a comment through the webinar chat function, although no public comment period is scheduled
- A meeting summary and materials will be made available on PCORI's website following the meeting
- Visit [www.pcori.org/events](http://www.pcori.org/events) for more information on future activities

# Welcome

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**Kristin Carman**

Director, Public and Patient Engagement

**Dave White**

Chair, Advisory Panel on Patient Engagement

**Tom Scheid**

Co-chair, Advisory Panel on Patient Engagement



# Presenting speakers

## Day One



- **Gwen Darien**  
Executive Vice President, Engagement and Patient Advocacy, Patient Advocate Foundation and National Patient Advocate Foundation
- **Kristin Carman**  
Director of Public and Patient Engagement, PCORI
- **Lia Hotchkiss**  
Director of Engagement Awards, PCORI
- **Bill Silberg**  
Director of Communications, PCORI
- **Hal Sox**  
Director of Peer Review and Scientific Publications, PCORI
- **Carly Parry**  
Senior Advisor, Care Coordination and Transitions Research Initiatives, PCORI
- **Maureen Maurer**  
Principal Researcher, American Institutes for Research

# PEAP Advisory Panel Members



- **Dave White - Chair**  
*National Committee for Quality Assurance*
- **Tom Scheid – Co-Chair**  
*Patient Advocate*
- **Jennifer Canvasser**  
*Necrotizing Enterocolitis Society*
- **Katherine Capperella**  
*Johnson & Johnson*
- **Anita Roach**  
*National Sleep Foundation*
- **Jill Harrison**  
*Brown University*
- **Matthew Hudson**  
*Greenville Health System*
- **Freddie White-Johnson\***  
*University of Southern Mississippi*
- **Gwen Darien**  
*National Patient Advocate Foundation*
- **Sonya Ballentine**  
*Illinois Institute of Technology College of Psychology*
- **Marilyn Geller\***  
*Celiac Disease Foundation*
- **Sarah Donelson\***  
*BioMarin Pharmaceuticals*

*\*Unable to attend*

# PEAP Advisory Panel Members



- **Beverly Rogers**  
Bev J Rogers Enterprises, LLC
- **Maureen Fagan**  
University of Miami Health System
- **Brendaly Rodriguez**  
University of Miami, and FL Community Health Worker Coalition
- **Umair A. Shah**  
Harris County Public Health
- **James Harrison**  
University of California San Francisco
- **Emily Creek**  
Arthritis Foundation
- **Megan Lewis**  
RTI International
- **Ting Pun**  
Patient Advocate
- **Jack Westfall**  
University of Colorado School of Medicine
- **Norah Schwartz**  
El Colegio de la Frontera Norte
- **Sandy Sufian**  
University of Illinois at Chicago, College of Medicine

# Introductions

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Advisory Panelists





# SPOTLIGHT: PEAPS IN ACTION

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## Gwen Darien

Executive Vice President, Patient Advocacy  
and Engagement Patient Advocate Foundation and  
National Patient Advocate Foundation

# **“What Matters to You?”**

## **Ask, Listen, Act**

### **Perspectives on NPAF and PAF’s Patient Advocacy and Engagement**

**PCORI PEAP Meeting**

**June 27, 2019**

**Gwen Darien**

**Executive Vice President, Patient Advocacy  
and Engagement**

**Patient Advocate Foundation and  
National Patient Advocate Foundation**

## **Patient Advocate Foundation:**

One Patient at a Time, One Community at a Time

## **National Patient Advocate Foundation:**

Working at the Intersection of Direct Patient Services and System Change



# Ask, Listen, Act



# Person-Centered Care and Health Care Delivery Systems


Core beliefs

**To fulfill our commitment to equitable access to affordable quality health care, we believe:**

- Patients play a key role in driving systemic changes—beginning with self-advocacy, extending to active efforts to educate other key stakeholders
- Patients are part of the change process, not simply objects of that change
- Patients and their providers co-create health and health care plans that meet clinical objectives, life goals and individual values

## Foundation of Patient Advocacy and Engagement

Build upon what we've learned, what patients need to know

- **Elevate** issues of PAF's patient populations and solutions of PAF's case management
  - **Integrate** patient life experience and social and political context
  - **Iterate** by implementing a learning system—feedback loop, evaluation and continual improvement
- 



# The Roadmap to Consumer Clarity in Health Care Decision Making

## Making Person-Centered Care a Reality

### What is the Road Map to Consumer Clarity?


The Roadmap to Consumer Clarity in Health Care Decision Making proposes actionable models to drive person-centered care at key decision points for people facing or living with serious illnesses.



## **Moving the Roadmap Forward**

### **Skilled Communications Workshops**

Skilled communications are at the heart of co-creating health for both patients and their health care team. Working with community groups, offered a series of Skilled Communications Workshops for the populations they serve.

- Telling your story—both at the individual and collective level
  - Highlighting issues specific to those communities and programs that are working to address these challenges and advance person- centered care
  - Providing an opportunity for participants to learn valuable skills while engaging in open, interactive discussions about what matters to them
  - Commitment to making the Roadmap model of shared decision making a reality
- 



## Cost of Care Conversations

### Raising Awareness of Financial Toxicity in Health Care and Bringing Cost of Care Discussions to Advocates, Patients and Caregivers

- Financial Toxicity and Cost of Care Discussions: Getting the message to key stakeholders
- Pilot projects to advance this agenda. Focus on patient experience, utilizing case studies and best practices
- Elevate the issues and solutions of PAF's patient population
  - Talking about Cost of Care website
  - Webinar series
  - Fall Policy Consortium
  - Talking about Cost of Care: A Guide for Case Managers and Patient Navigators

# Understanding Tradeoff's

## Real world impact of cost of care



# Closing the Gap Between Cost of Care Conversations and Talking about Money

## Transparency and Taboos



# No Shame in the Game

## Addressing Barriers to Talking About Money

“I finally realized that I needed to speak up when I was in my doctor’s office. I needed not to wait until I got to the pharmacy and found out my prescription was going to be \$100—and I didn’t have it. Then I was ashamed and might just walk out, or not take the medicine, and not tell my doctor. What I learned is to speak up, tell the doctor about my financial concerns. There’s no shame in my game.” Shirley Bridgett, Heart Patient, Mississippi



# Too expensive to live....


## Impact of financial distress

“The bills started to mount up, forty, sixty, eighty thousand dollars and I basically started to think, I can’t afford to live, and I decided to stop treatment.” Tom Ema, cancer patient



## Five Key Takeaways


### Integrating Cost of Care Conversations into Shared Decision Making and Care

1. Patients and caregivers want to talk about the costs of their care but face a range of barriers in having these conversations
  2. The financial impact of care includes the direct costs of that care but also extends to indirect and life-style related issues
  3. Physicians and providers are increasingly aware of the need to discuss costs and willing to do so, but often lack the training and tools to have these conversations
  4. Cost of care conversations are more likely to happen when physicians initiate them—and generally do not take very long
  5. The key to assuring that costs of care conversations occur is “normalizing,” them—developing the procedures and systems to make these discussions part of the shared decision-making process
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# Barriers and Solutions to Accessing Genomic Medicine:

## Realizing the Benefits of Genomic Medicine for All

NPAF Spring 2019 Policy Consortium

- Innovative ways of raising awareness of the potential for genomic medicine to improve individual and population health and alleviate barriers to access
  - Preliminary results of a study for which we partnered with Vence Bonham, JD, Senior Advisor to the Director on Genomics and Health Disparities at NHGRI, on barriers and disparities to access to genomic medicine
  - Bring the patient and provider voices to the table on this important topic
  - Explore challenges that patients, their caregivers and health care providers face in assuring equitable access to genomic medicine
- 



# The Case Manager Experience

Evaluating Underserved Populations' Access to Genomic Medicine





# Closing the Gaps

Personal and Cultural Barriers to Genomic Medicine What does our genome say about us? Is it true? Who does it benefit?



# What do Patients Say?

Putting Narratives at the Heart of Communications “I want to be treated like a ‘beating heart,’ not a ‘sick breast.’”



## On the Front Lines

PAF Case Managers Share Their Perspectives Achieving equity in genomic medicine so that “angels” don’t have to check obituaries





# This Is Us

At the NPAF Fall 2019 Policy Consortium



# BREAK

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We will return at approximately  
11:00am EDT

# ENGAGEMENT PROGRAM UPDATES

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# Public & Patient Engagement

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**Kristin Carman**

Director, Public & Patient Engagement



# Pipeline to Proposal Awards Initiative: Evaluation Findings

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**Lia Hotchkiss**

Director, Engagement Awards





# Pipeline to Proposal Program (P2P)



- Established in 2013 to support stakeholder partnerships focused on health issues affecting their communities with the aim of increasing and advancing PCOR in their communities and beyond.
- Program Structure:
  - Five Pipeline Award Program Offices (PAPOs) contracted with awardees and provided technical assistance
    - Health Resources in Action (Boston, MA)
    - Michigan Public Health Institute (Lansing, MI)
    - Georgia Health Policy Center (Atlanta, GA)
    - The National Network of Public Health Institutes (Washington, DC, and New Orleans, LA)
    - Trailhead Institute (Denver, CO)
- Awards:
  - Tier I (Tier A): Seed money to individuals and groups with healthcare research ideas and interest in PCOR
  - Tier II (Tier A): Develop research capacity, create new partnerships, and build infrastructure needed to conduct research
  - Tier III (Tier B): Develop high-quality research proposals that can be submitted for PCOR funding

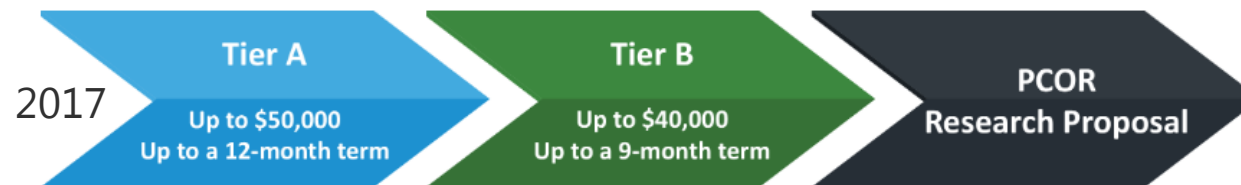
# P2P Awards



**Cycle 1**      30      27      22

**Cycle 2**      47      44      42

**Cycle 3**      46      41      0



**Cycle 4**      50      0



# P2P Program Goals



1. Strengthen researcher-patient-stakeholder relationships, particularly in underrepresented communities
2. Build partnership capacity for PCOR funding
3. Engage partnerships in the research process
4. Successfully establish infrastructure for patients, caregivers, and other stakeholders to increase knowledge about PCOR and engagement in research, dissemination, and implementation

\*Many of P2P's awardees were grassroots efforts new to PCOR and PCORI

# P2P Evaluation

In 2018, PCORI contracted with NORC to conduct an evaluation of P2P.

The evaluation answered three key research questions:

1. To what extent did P2P achieve its intended goals?
2. What were the unanticipated positive or negative outcomes of P2P?
3. How can PCORI improve P2P or other similar programs that may build on P2P's progress in the future?

# Data Sources



- Primary
  - Semi-structured 60-minute telephone interviews with P2P and other stakeholders (n=87) conducted October 2018 - January 2019
- Secondary
  - P2P applications submitted from 2013-2017
    - Funded applications (n=177)
    - Unfunded applications (n=809)
  - P2P final reports submitted at end of each tier (n=351)\*
  - Learning About Partnerships (LEAP) survey completed surveys from P2P partners and project leads (n=310)
  - PCORI administrative data on awardee location, target population, and disease/condition focus

*Note: \*Two awardee reports were missing from the sample*

# To What Extent Did P2P Achieve its Intended Goals?

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# To What Extent Did P2P Achieve its Intended Goals?

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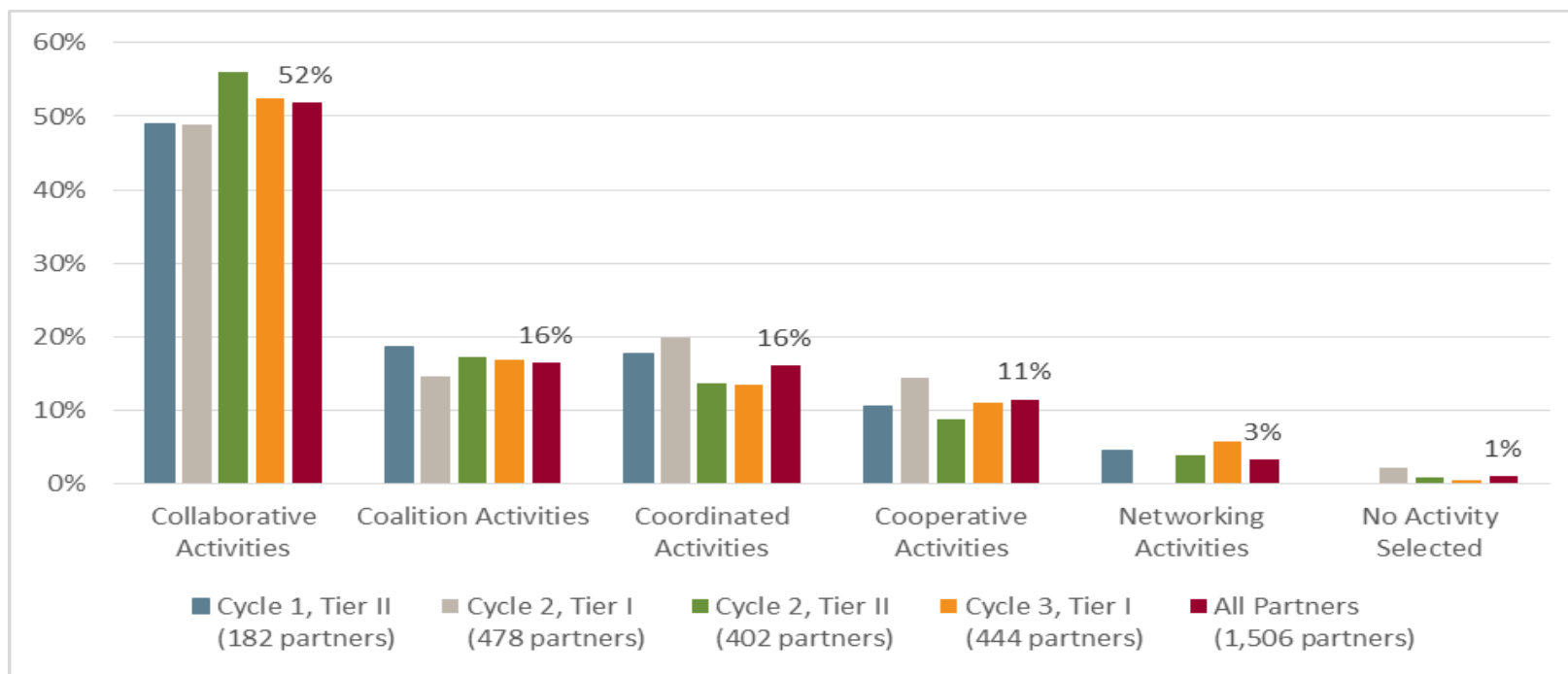
Goal 1: Strengthen Researcher-Patient-Stakeholder Relationships



# Researcher-Patient-Stakeholder Relationships



- In final reports, awardees described each partner's activities in the partnership (1,506 Partners, 127 awardees)



Data source: Awardee final reports. Notes: Fifteen partners among reporting awardees were not assigned an activity type by awardees and are not illustrated in this analysis but counted in the denominator of percentage of partners per activity type. Cycle 1 awardees could not provide information about more than 10 partners in final reports. Percentages are rounded,  $\geq 0.5$  percentage point rounded up to the nearest whole percentage point,  $\leq 0.49$  percentage point rounded down to the nearest whole percentage point.



# Inclusion of Underrepresented Populations

- PCORI sought to incorporate underrepresented communities and stakeholders that may not be traditionally involved in the research process

Partner Types, n=127

Partner Types	Number of awardees (Share of Awardees, %)
Researcher	112 (88)
Patient/consumer	108 (85)
Clinician	86 (68)
Patient, consumer, or caregiver advocacy organization	76 (60)
Caregiver or family member of patient	69 (54)
Clinic/hospital/health system	60 (47)
Community-based organization	40 (32)
Policymaker	25 (20)
Subject matter expert	20 (16)
Training institution	10 (8)
Payer	7 (6)
Life sciences industry	6 (5)
Other	73 (57)

Note: Categories provided by PCORI in final reports

Health Issue of Focus*	Awardee Count	Awardee Percent of 177 Awardees
Chronic conditions	66	37%
Mental and behavioral health	48	27
Diabetes	26	15
Cancer	23	13
Rare diseases*	20	12

- Most awardees focused on adults (55%), seniors (46%)
- Some targeted racial and ethnic minority populations
  - African American (58 awardees, 33%)
  - Hispanic/Latino (56 awardees, 32%)
  - Asian (19 awardees, 11%)

Data sources: Awardee final reports (Cycle 1, Tier II only), PCORI administrative data. Notes: Awardees may have changed disease focus over time and therefore may be counted multiple times. Percentages are rounded. Denominator for percent is 177 awardees.

# **To What Extent Did P2P Achieve its Intended Goals?**

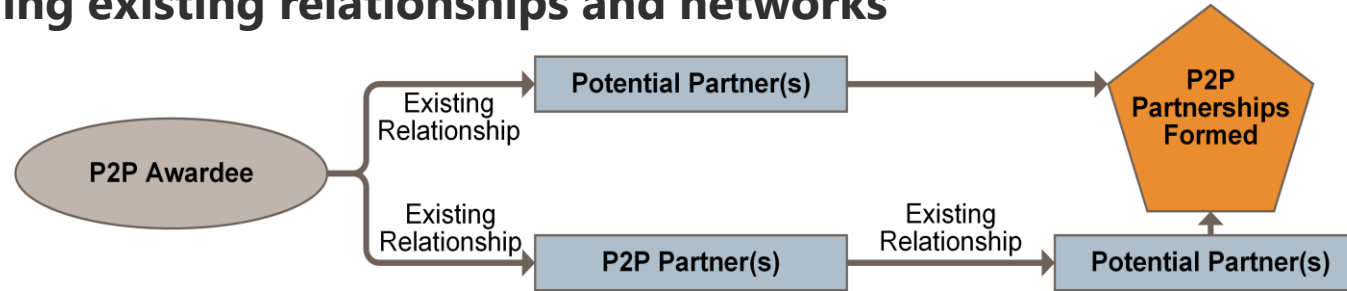
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Goal 2: Build Partnership Capacity for PCOR  
Funding

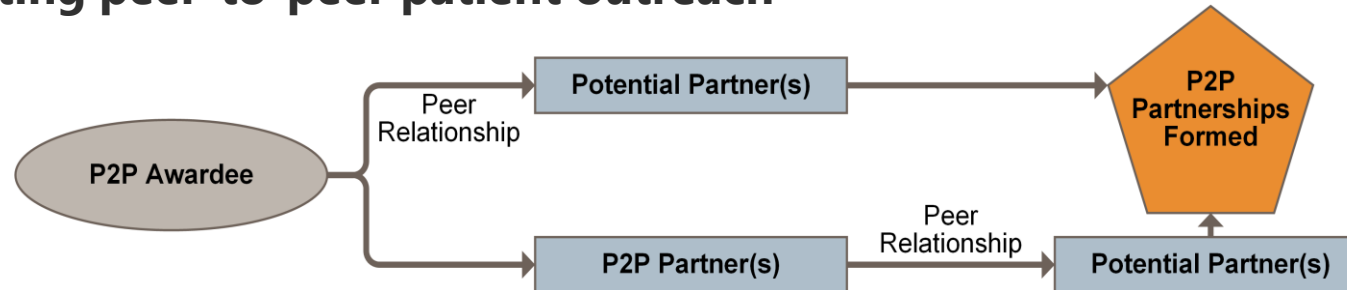


# Common Partner Recruitment Approaches

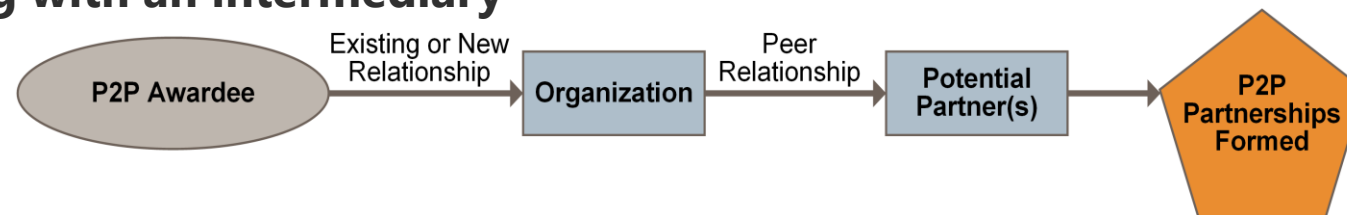
## Leveraging existing relationships and networks



## Conducting peer-to-peer patient outreach



## Working with an intermediary



# Promising Partner Recruitment Practices



Having a **direct connection** to the target population or community served



Leveraging **social media**, particularly to recruit patient partners



**Tailoring** recruitment approaches for different types of stakeholders



Identifying individuals or organizations **who had a stake** in a specific health topic or disease focus

*"In the past, I tried advertising in newspaper, .... That doesn't work out because you get responses, but they might not be [from people who are] embedded in the community, and [these individuals] won't be as successful as someone who is well known in the community."*  
– P2P Awardee

# **To What Extent Did P2P Achieve its Intended Goals?**

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Goal 3: Engage Partnerships in the Research  
Process



## Build Transparency, Honesty, Trust

- Establish a **flat partnership structure**
- Provide opportunities for partners to **provide feedback**
- Meet in a **neutral setting** (i.e., outside of a medical setting)
- Use **breakout groups** during meetings

*"...when I engage with my clinicians it's always through a portal or some sort of approved, compliant communication vehicle, but I can talk, text, direct message people from the [P2P] project. So, I think that implies some level of trust that is deeper than some regular engagements that we have in the healthcare space." – P2P Project Partner (Interview)*

*"As a partnership we have learned about group dynamics, coalitions, networking and about strategies to activate patients and their families so they can effectively work close with researchers and stakeholders. We have been able to give patients a voice in forums usually not open to them...patients have taught us humility and the importance of respect and flexibility when working with them." – P2P Awardee (Final Report)*

## Foster Reciprocal Relationships

- Carve out **defined roles** for partner (e.g., facilitating meetings, leading recruitment activities)
- Create opportunities for partners to **leverage their skills sets**
- Use **meeting facilitators** to strengthen communication and neutralize power dynamics

*"...the researchers and clinicians really had to sit down and listen and give patients an equal voice in the project decisions." – P2P Partner (Interview)*

*"... We would go into these meetings with our advisory board with a pre-set agenda ... so once we totally handed over the reins [to parent partners], they took us in a direction that spoke to the needs of the community." – P2P Awardee (Interview)*

*"You challenge people a little bit but not challenge them so much that they felt like they didn't have a voice." – P2P Partner (Interview)*



## Value Partnerships

- **Compensation** for partners can include financial and non-financial incentives (e.g., meals, honoraria, gift cards)
- **Emphasize goals** of the partnership through incentive design
- Think of engagement as a **long-term investment** over the course of the project

*"I think it did help that when we had in-person meetings, we were compensated for our time. And in the evenings or lunchtime there was food. That extra bit to show appreciation was a plus." – P2P Partner (Interview)*

*"...particularly in Native American communities, [partners] ...expect to be fairly compensated for their time and expertise. The funding support allowed us to bring people together in a respectful way, demonstrating that we valued their experience and expertise as partners." – P2P Awardee (Final Report)*

## Engage in Co-Learning

**Provide a platform** for partners to tell their own stories without a specific framework or agenda, making sure stories are still **meaningfully connected** to the project

*"With respect to the researchers, they were so amazed that they were getting feedback they had never heard [and] they just wanted to keep participating." – P2P Partner*

*"An interesting change was [that] clinicians were excited that patients were equal members of the stakeholder group and it [was not] tokenism. We're kind of breaking down stereotypes about what patients can do." – P2P Partner*

*"In meetings we also encouraged [patients] to share specific stories that related to the research we were doing. Then people in their group shared stories...we got a personal look on what the research meant on an individual level. We wanted our patient stakeholders to feel like they could elevate their experiences." – P2P Awardee*

# To What Extent Did P2P Achieve its Intended Goals?

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Goal 4: Successfully Establish Infrastructure to Increase Knowledge about PCOR and Engagement in Research, Dissemination, and Implementation



# P2P Outcomes



## Short-term Outcomes

- **Increased awareness** and conducted outreach to underserved communities about health issues of interest (81% of awardees), created tangible outputs (62%)
- **Acquired new knowledge** of CER and PCOR
- **Applied Engagement Principles** to pre-research activities

## Intermediate Outcomes

- 70% of awardees reported **increased capacity** for partnerships to engage in and conduct research
- Awardees were **more likely to engage in PCOR** (96%, 152 awardees reporting)
- Awardees felt **more prepared to pursue research funding** (Cycle 1 Tier I: 100%; Cycle 1 Tier II: 69%; Cycle 2 Tier II: 84%; Cycle 3 Tier I: 91%)

## Long-term Outcomes

- 151 of 170 awardees planned to **pursue funding** from PCORI or other funders in their final reports
- According to PCORI administrative data and final reports:
  - 1 P2P awardee **received funding for CER**
  - **27 were awarded Eugene Washington PCORI Engagement Award funding (16%)**
  - **12 awardees received funding from other sources:** 5  
from a hospital/university, 4 from a foundation; 1 from a government entity; one from private donation; and one unspecified.
- In final reports and during interviews, awardees described desire to **sustain partnerships** after P2P

# P2P Challenges



- P2P awardees experienced challenges
  - Time and resources
  - Conducting work in a new way—with patients and other stakeholders, rather than researchers, driving the process
  - Changes in award and program structure (2017)
  - Need for greater technical assistance
  - Areas stakeholders identified for research were exploratory or lacked evidence base/comparators, not ideal for CER\*

\*Mid-way through the program, PCORI modified program requirements to allow awardees to explore opportunities beyond developing proposals for CER. Awardees pursued other research opportunities, developing proposals on stakeholder engagement, etc.

# What Are Some Examples of Unanticipated Outcomes of P2P?

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# Examples of P2P Unanticipated Outcomes



## Unanticipated Outcomes

- Changed **clinical care** and **research practices** (8%): new screening tools, new ways of documenting conditions in health records, and implementing new provider trainings based on P2P experiences
- P2P awardees **pursued other sources of funding** beyond PCORI, including from foundations, government entities; and universities or university-affiliated hospitals.

# How Can PCORI Improve P2P or Similar Programs in the Future?

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# Considerations for Future Program



- Awardees and partners needed a great deal of support, training, and technical assistance
  - Most helpful:
    - PAPOs worked synergistically with awardees
    - P2P Awardee Convention
    - Networking opportunities among awardees
  - Need more:
    - Examples of successful proposals
    - Guidance/assistance with developing CER proposals
    - Help with engaging patients
- Longer tier time frames
- Increased funding
- Reduced financial reporting requirements (awards were cost reimbursement contracts)

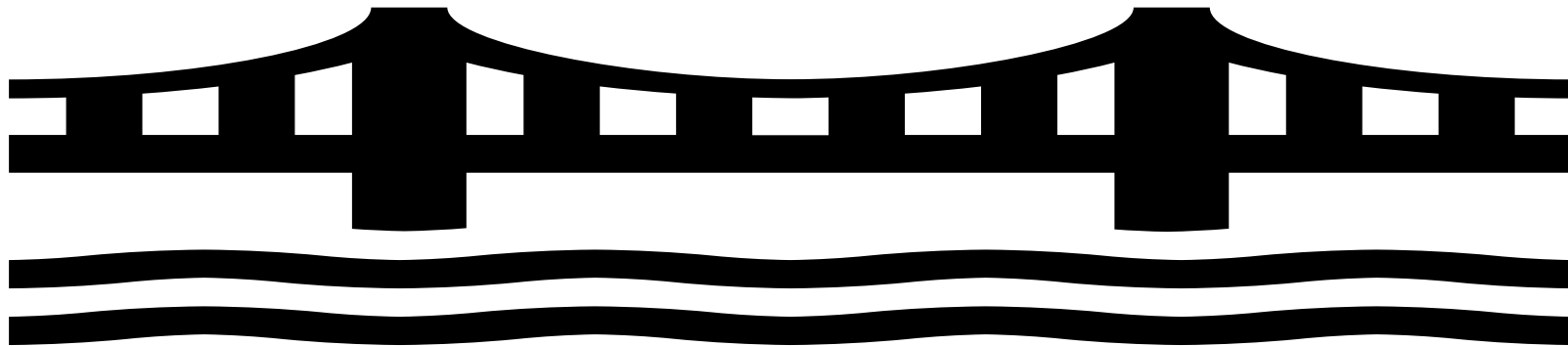
# Considerations for Future Program



- Ask applicants to more fully describe their research topic or question so projects with greatest potential for PCOR are awarded
- Applications and final reports for multi-tier, multi-cycle awards should contain a core set of the same questions, potentially asked at different times in the program to record project evolution
- Ask all awardees and partners to complete the LEAP survey or similar instrument about partnerships
- Provide enhanced technical assistance including:
  - Regular updates on PCORI's research priorities
  - More training on CER
  - Additional opportunities to consult with PCORI Science Team
- Create opportunities for P2P graduates to participate in future PCORI programs
- Consider additional ways to help P2P graduates become more competitive among experienced researchers

# Key Lessons Learned

- Diversity of awardees and partners suggested P2P achieved representation from stakeholders not typically involved in CER or PCOR
- Engaging stakeholders who often lacked experience in conducting PCOR was challenging
- Requires culture change, takes time
- Challenges highlight the importance of learning network or tailored technical assistance to meet individual awardee and partnership needs



# Key Lessons Learned

- P2P provided the **funding, training and opportunity** for stakeholders to learn *how* to engage partners in pre-research
- P2P created a **multi-stakeholder environment** to conduct pre-research
- P2P partnerships **successfully engaged underrepresented stakeholders** in communities across the country
- **Sustainability was a focus of P2P**
  - PCORI and PAPOs worked with awardees to develop deliverables to facilitate sustainability beyond P2P (e.g., governance documents, communication and sustainability plans)



# For Discussion

- How can PCORI support early partnership development? What types of activities should we fund?
- How can PCORI successfully engage with and encourage applications from underrepresented stakeholder communities and institutions that serve them from across the country?



# **BREAK**

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We will return at approximately  
12:45pm EDT

# PCORI 2019 ANNUAL MEETING

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***Making a Difference: Using Patient-Centered Research Results in the Real World***

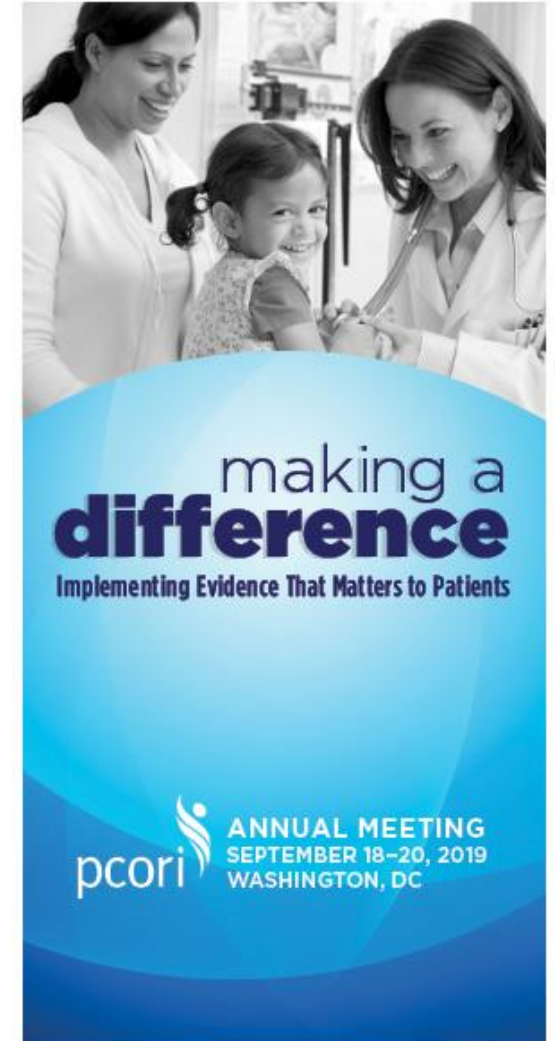
**Bill Silberg**

Director, Communications



# General Overview

- Dates: September 18-20, 2019
- Venue: Washington Marriott Wardman Park, Washington, DC
- Theme: *Making a Difference: Using Patient-Centered Research Results in the Real World*
- Goal: Report to the nation on PCORI's progress in funding research to determine which care approaches work best, for whom, and under what circumstances, with a focus on outcomes important to patients.
- Up to 1,000 members of the healthcare community
  - Largest attendee groups in the past: Researchers (~40%); Patients and caregivers (~35%); remainder a mix of clinicians, hospitals, health systems employers, insurers. Another 500+ attend via webcast.
- >490 registrants to date



## Opening Keynote:

- Drs. Scott Berns and Leslie Gordon, Progeria Research Foundation

## Plenary topics:

- What's Right for Me? Practical Approaches to Personalized Medicine
- How Can We Do That? Effectively Putting New Evidence into Practice
- How Engagement is Making Research More Useful
- What's Next? The Future of Patient-Centered Outcomes Research

# Breakout Sessions



## Breakout session topics:

- Dissemination and implementation
- Engagement
- Disparities/equity
- Pain care/opioids
- Chronic conditions
- Telehealth
- AHRQ's support of uptake of evidence
- PCORnet
- Patients within the Medicaid system
- Serious mental Illness
- Veterans health care
- Shared decision making
- Methods
- More effective diagnosis/surveillance

# Questions?

# PEER REVIEW AT PCORI

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## *A Model of Trustworthy Comprehensive Reporting of Research*

**Hal Sox**

Director, Peer Review and Scientific Publications





# Promoting Our Model of Peer Review in JAMA



More ▾

This Issue

Views **4,198** | Citations **0** | Altmetric **23**

Viewpoint

March 28, 2019

## A Model for Public Access to Trustworthy and Comprehensive Reporting of Research

Marina Broitman, PhD<sup>1</sup>; Harold C. Sox, MD<sup>1</sup>; Jean Slutsky, PA, MSPH<sup>1</sup>

» Author Affiliations | Article Information

JAMA. 2019;321(15):1453-1454. doi:10.1001/jama.2019.2807

FREE



Prof. Karen Woolley

@KWProScribe

It must be done - by law! @PCORI posts plain language peer-reviewed abs in public repository. 160-255 hrs for lx to write full report; 8-9 mths to peer review it (w #PPI - relevance, use), 90d to post abstract. ROI? <https://t.co/P71DTxz038> #GPP4

03 Apr 2019

## PCORI's Commitment to Publicly Accessible Study Results Captured in JAMA Viewpoint

Date: March 28, 2019

Blog Topics: [Evaluating Our Work](#), [Research](#)

At PCORI, we're committed not only to funding research that can help patients and those who care for them make better-informed healthcare decisions, but to doing all we can to see that the results of those studies are made widely available. As we outline in a [newly posted JAMA Viewpoint](#), PCORI's approach to this is unique not just among research funders but across the healthcare community more broadly.



Diana James

@dianalouisej

For those with an interest in free to view peer reviewed research, this is very interesting. <https://t.co/MOSzK1ki0N>

28 Mar 2019



TrialScope

@TrialScope

In a first for a US-based research funding organization, in 2010 Congress required the Patient-Centered Outcomes Research Institute @PCORI to make research results publicly accessible within 90 days of their receipt. See where we are today: [@JAMA\\_current h](#)

28 Mar 2019

# Presentation Outline

- **Background: how medical journals work**
- Origins of peer review at PCORI
- Process of peer review
- Challenges for PCORI: timeliness vs. quality
- Progress to the present
- Dissemination
- Benefits and Downsides
- Summary

# How a Medical Journal Works

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Annals of Internal Medicine

# Annals of Internal Medicine (as of 2008)

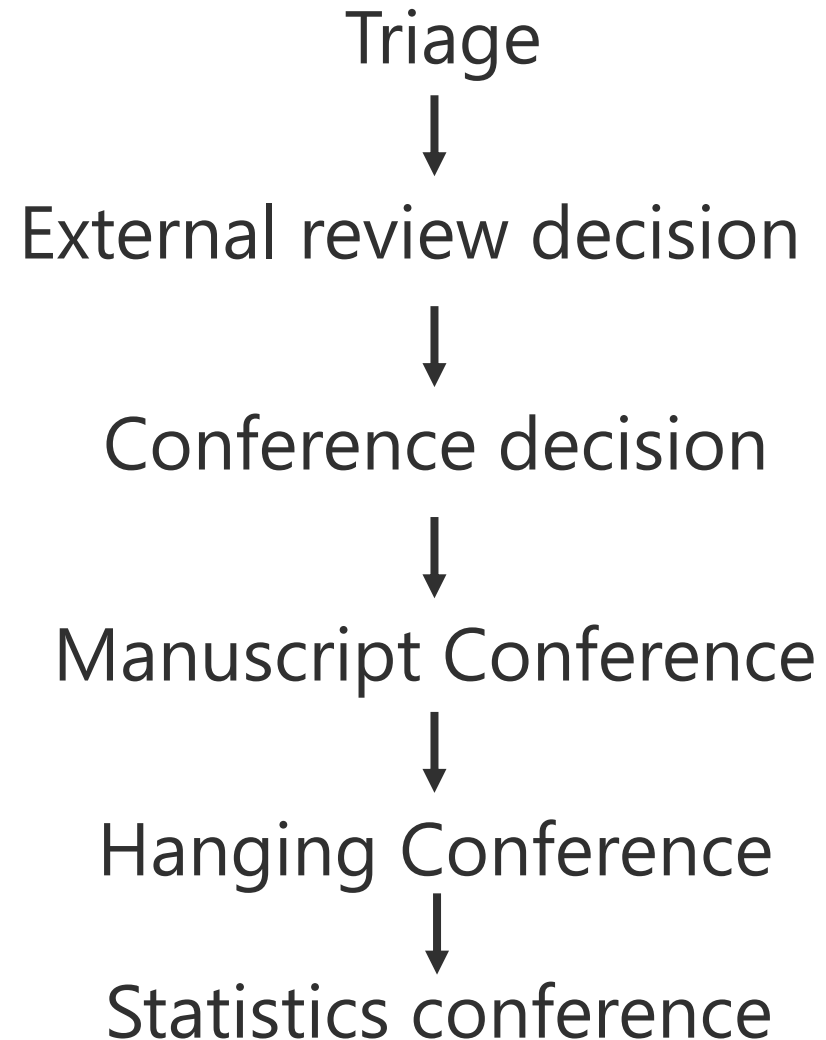


- 87,000 subscribers
  - The largest specialty journal
  - International readership
- Impact factor 16.25:
  - Ranks fifth among all clinical journals (NEJM, JAMA, and Lancet lead)
- 2800 manuscripts per year
  - 30% from abroad
  - Accept 6% of original research articles

# Annals Editorial Staff

- Senior Editors: 5.5 FTE
  - Editor-in-chief 1.0
  - Executive Deputy Editor 1.0
  - Deputy Editor for e-publication 0.6
  - Deputy Editors 2.9
- Associate Editors: 10 x 15%
- Statisticians: 5 (1.8 FTE)
- Managing Editor
- Manuscript representatives: 3
- Others: 2

# The Review Process at Annals



# Presentation Outline

- Background: how medical journals work
- **Origins of peer review at PCORI**
- Process of peer review
- Challenges for PCORI: timeliness vs. quality
- Progress to the present
- Dissemination
- Benefits and Downsides
- Summary

# The Beginnings: Mandates of the Legislation



- Report all research results
- Peer review the results
- Assess their scientific integrity
- Report study limitations and efforts to identify patient subgroups in which the comparative effectiveness of the study interventions differs from the entire study population..
- Report the results within 90 days of receipt



# PCORI's Response to the Legislative Mandate



- Report all research results
  - Require a final report structured like a journal article but covering all methods and results.
- Peer review the results
  - Pay a contractor to manage the external review process, which is structured like a journal's process.
- Assess their scientific integrity
  - Peer review → conclusions tempered by the evidence

# PCORI's Response to the Legislative Mandate

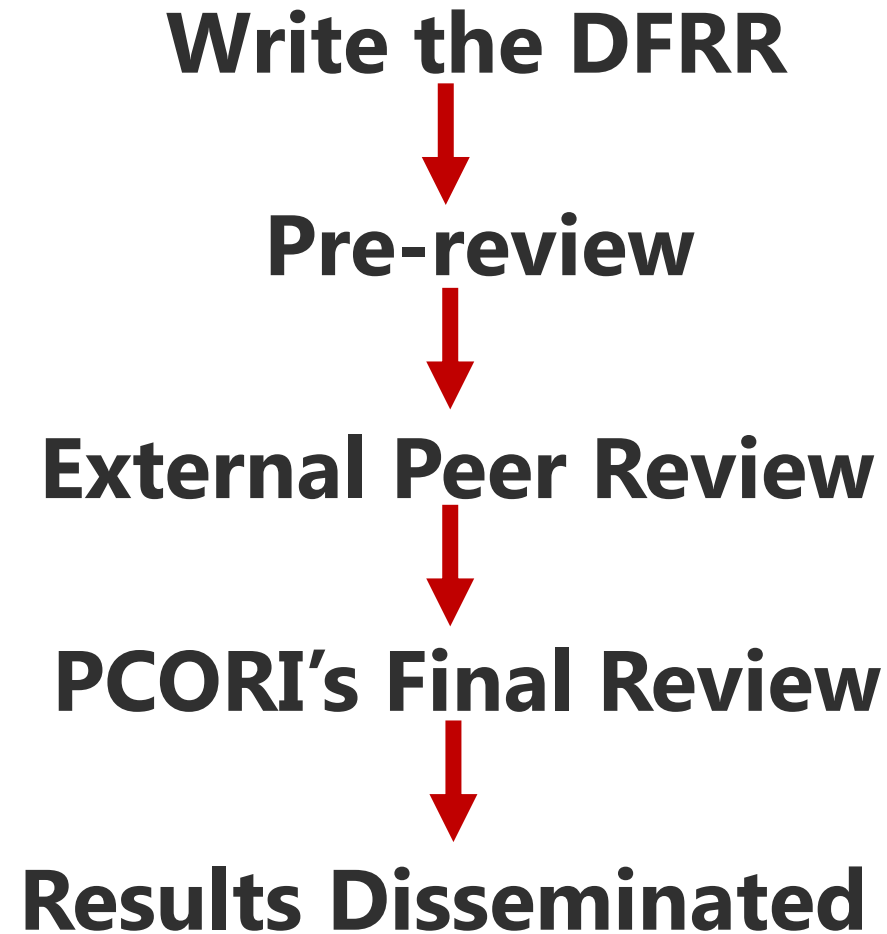


- Report study limitations and efforts to identify atypical responders to the study intervention →
  - required element in final report
- Report them within 90 days of receipt →
  - post abstracts—for the public and for professionals—based on peer-reviewed final report

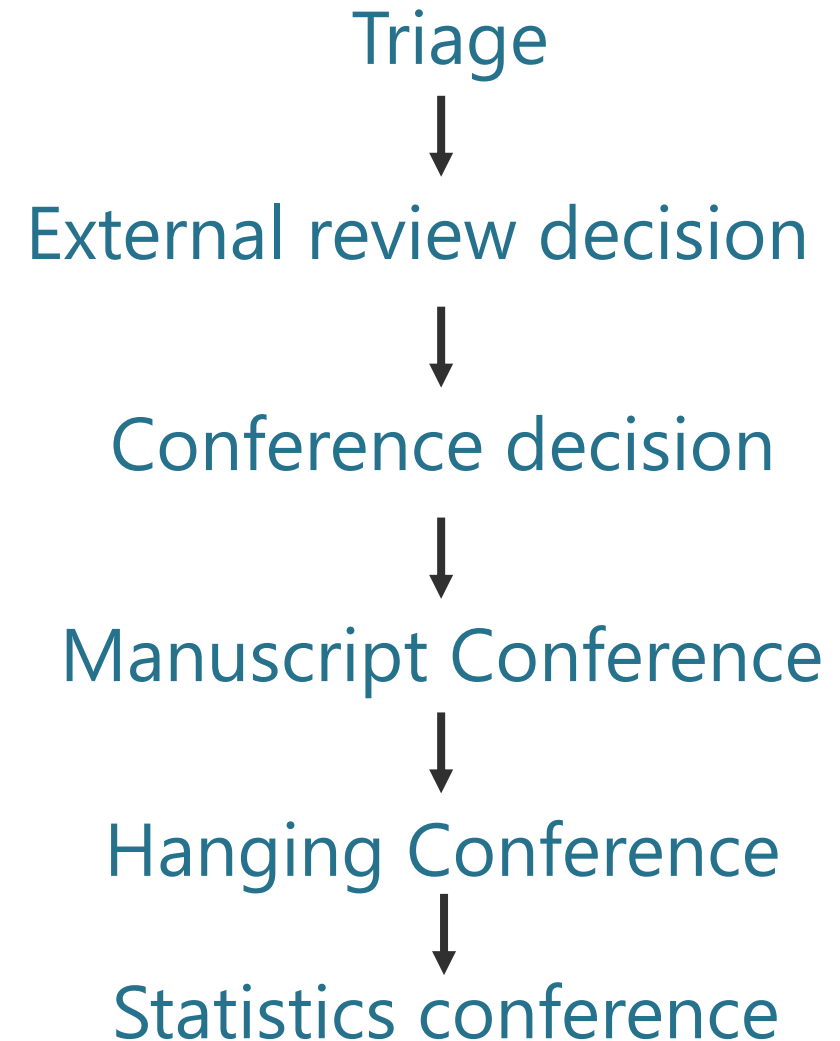
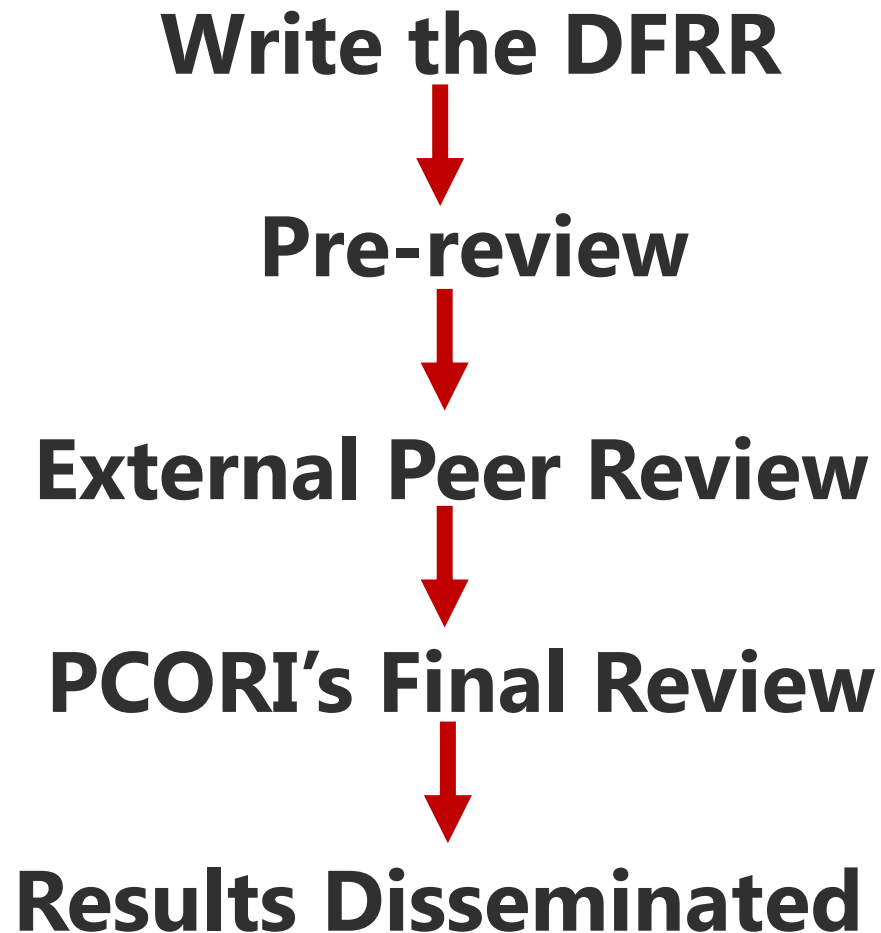
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# Peer Review Process for Awardees



# Peer Review Process for Awardees



	<b>Annals of IM</b>	<b>PCORI</b>
Percent Accepted	7%	100%
Volume of manuscripts per year	~3000	~100
Size of Typical Manuscript	3000 words	15,000+
Circulation	125,000 subscriptions	Posted on <a href="http://pcori.org">pcori.org</a> (open access)
Source of \$	Membership dues	The public

# Process: Journal vs. PCORI Peer Review



- **Main purpose of peer review**
- Journal: decision making → improvement of accepted manuscripts
- PCORI: improvement of all manuscripts → decision making about implementation
  - Scientific
  - Clarity
- **In common: assure scientific integrity**
  - Does the evidence support the study results?
  - Do the study results support the study conclusions?

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# The PCORI Challenge



- PCORI as an organization has two goals for peer review:
  - **Speed:** complete peer review quickly so that PCORI can post the results.
    - Getting results into the public domain quickly was an important goal of the drafters of the legislation that created PCORI.
  - **High quality:** the reports should be understandable and reflect accurately the scientific integrity of the results.
    - Both journals and PCORI want to establish and maintain a reputation for scientific integrity. Trustworthiness is all.

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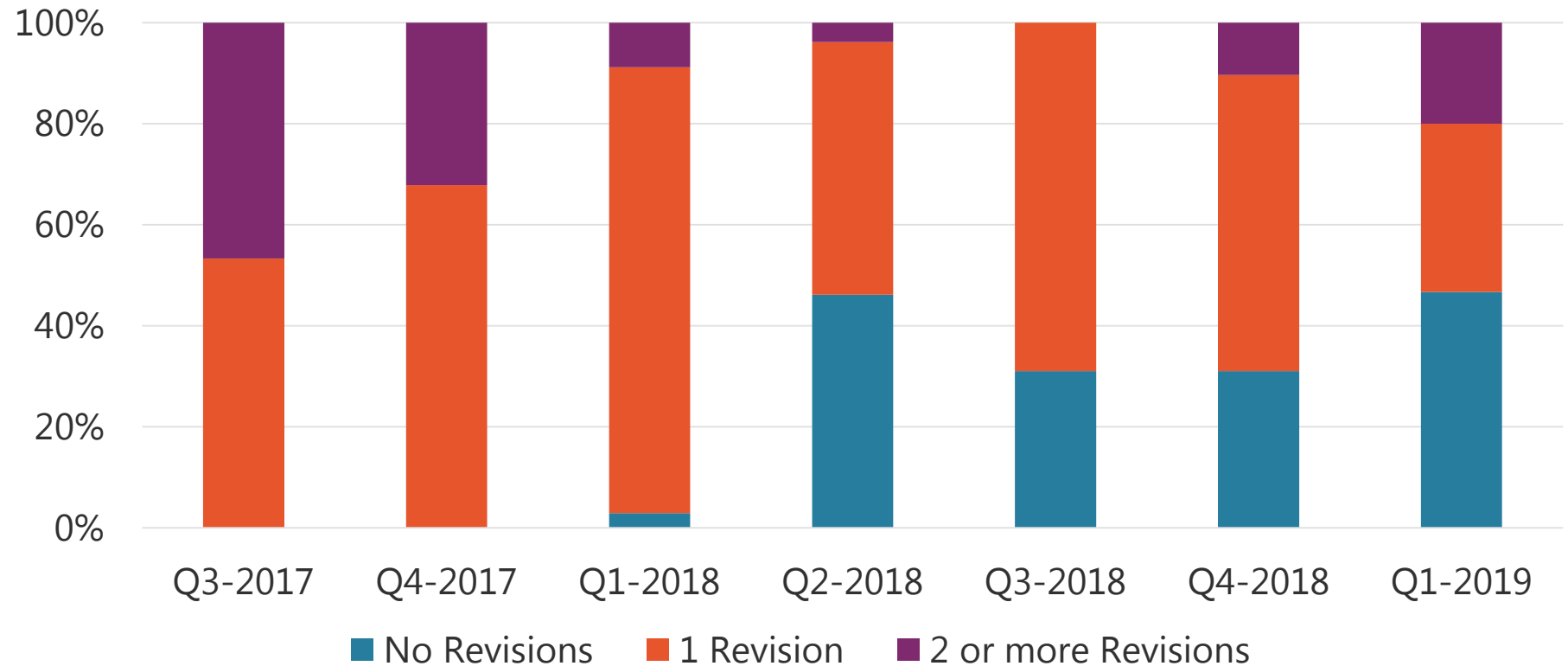
# DFRR Receipt, Review, & Posting (4/10/19)

DFRR count by current status	
Overall: DFRRs submitted to PCORI	288
DFRRs in pre-review (checked for clarity & completeness)	15
DFRRs in external peer review	42
DFRRs in final review (final read-through by Hal Sox)	14
FRRs accepted (PCORI has accepted, clock started for study abstracts)	217
FRRs & protocols posted, FRRs discoverable in Google Scholar	50

# Pre-Peer Review: Clarity and Completeness

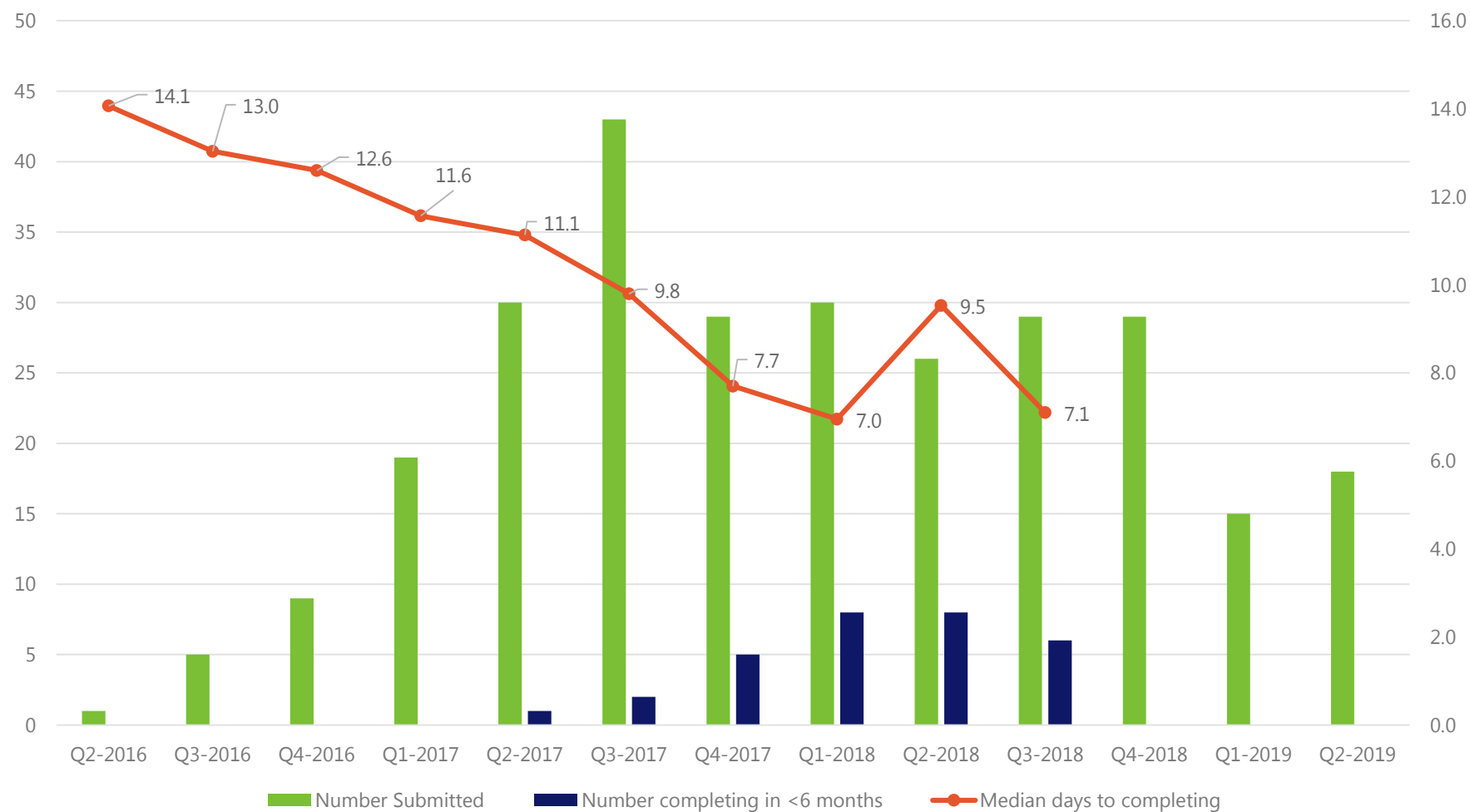
The pre-peer review phase was initiated because many DFRRs were coming in not following DFRR instructions, or poorly developed. This has changed with increased focus on the instructions and reminders from Program staff. More reports are coming in ready for peer review – no need for revisions before going external reviewers (blue).

Number of DFRR revisions before peer review, by submission date



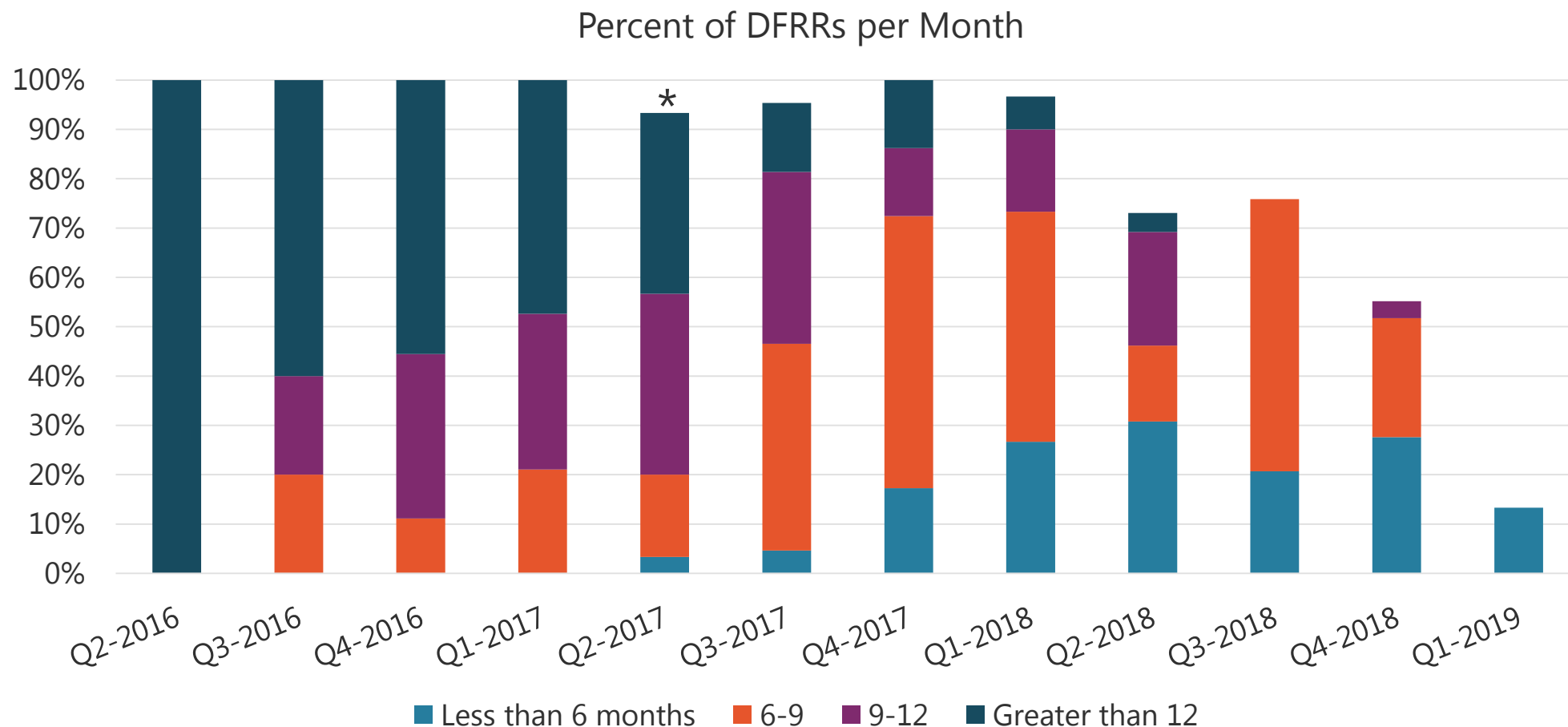
# Timeliness of Peer Review

In 2 years, we have cut the amount of time reports spend in peer review by half: 7 months from start to finish.



# Timeliness of Peer Review

The proportions are shifting – more of our reports are completing peer review in less than 6 months, and most in less than 9 months.



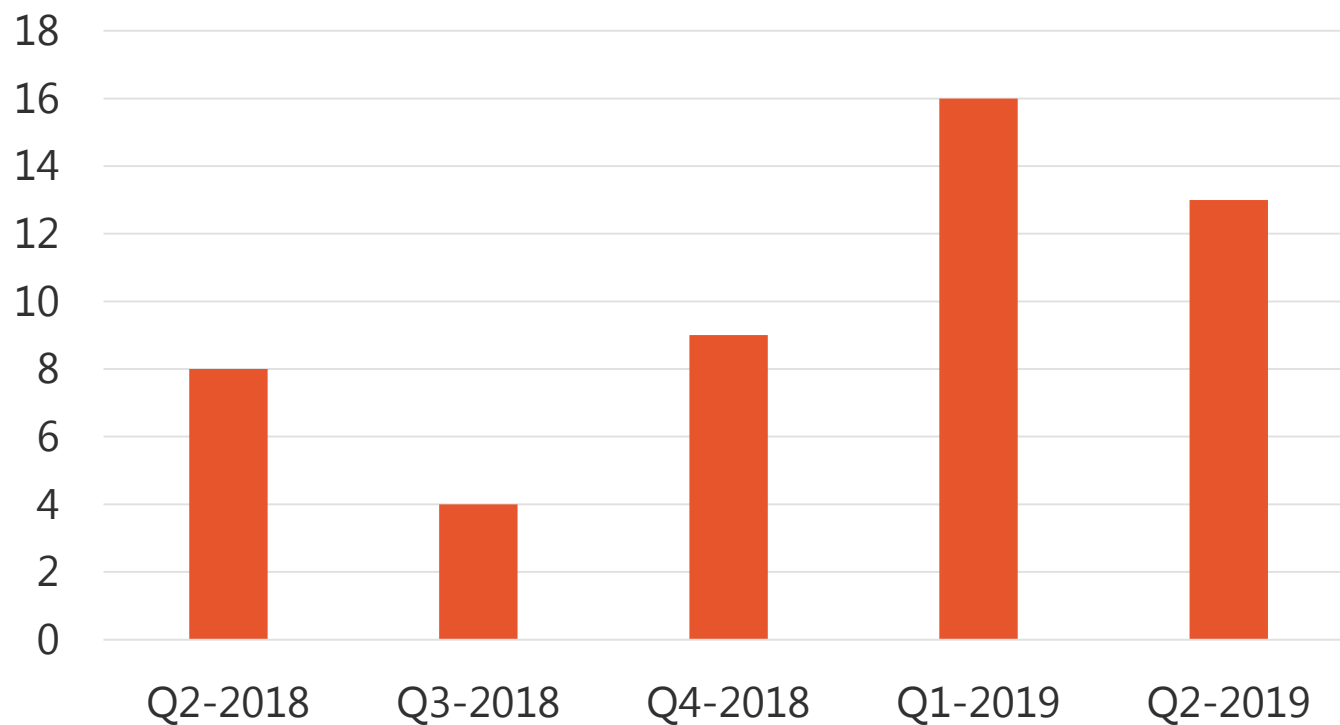
\* Incomplete bars indicate DFRRs submitted during that quarter that have not completed peer review.

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# Number of Final, Accepted Reports Posted to the Website, by Quarter



Up to 152 final reports may be posted by the end of FY2019

Average time from acceptance to posting: about 10 months

New FRR search function & presentation makes the reports easier to find



## Examining Health Outcomes for People Who Are Transgender

The objective of this study was to examine (1) the incidence of acute cardiovascular events and cancers and the prevalence of mental health conditions among transgender and cisgender people; and (2) perceived benefits of gender-affirming therapies such as hormones or surgery among transgender people.

Project page: [Examining Health Outcomes for People Who Are Transgender](#)

Principal Investigator: Michael Goodman, MD, MPH

Organization: Emory University

Original project title: Comparative Risks and Benefits of Gender Reassignment Therapies

HSRProj ID: HSRP20143115

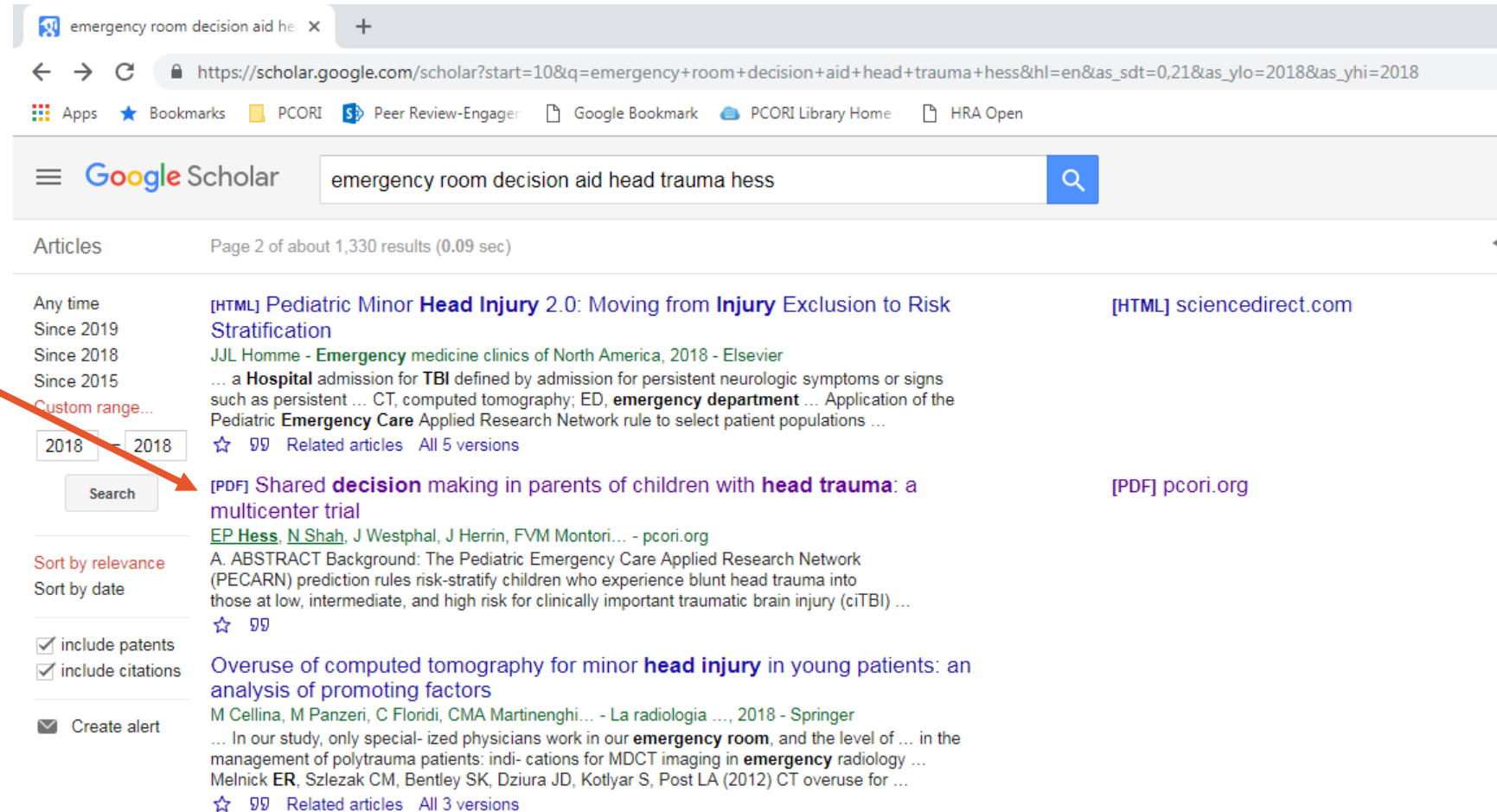
To cite this document, please use: Goodman M, Nash R. *Examining Health Outcomes for People Who Are Transgender*. Washington, DC: Patient-Centered Outcomes Research Institute (PCORI). <https://doi.org/10.25302/2.2019.AD.12114532>

[View the Final Research Report](#)



# Making Reports Publicly Available

- All final reports receive a DOI number, making them discoverable in Google & Google Scholar
- Final reports will also be posted on BookShelf, a searchable National Library of Medicine resource.



The screenshot shows a Google Scholar search interface. The search bar contains the text "emergency room decision aid head trauma hess". The results page shows "Page 2 of about 1,330 results (0.09 sec)". On the left, there are filters for "Any time", "Since 2019", "Since 2018", "Since 2015", and a "Custom range..." option with a date range of "2018 - 2018". Below these are checkboxes for "include patents" and "include citations", and a "Create alert" button. The search results list includes:

- [HTML] Pediatric Minor Head Injury 2.0: Moving from Injury Exclusion to Risk Stratification**  
JLL Homme - *Emergency medicine clinics of North America*, 2018 - Elsevier  
... a **Hospital** admission for **TBI** defined by admission for persistent neurologic symptoms or signs such as persistent ... CT, computed tomography; ED, **emergency department** ... Application of the Pediatric **Emergency Care** Applied Research Network rule to select patient populations ...  
☆ 🔍 Related articles All 5 versions
- [PDF] Shared decision making in parents of children with head trauma: a multicenter trial**  
EP Hess, N Shah, J Westphal, J Herrin, FVM Montori... - *pcori.org*  
A. ABSTRACT Background: The Pediatric Emergency Care Applied Research Network (PECARN) prediction rules risk-stratify children who experience blunt head trauma into those at low, intermediate, and high risk for clinically important traumatic brain injury (ciTBI) ...  
☆ 🔍
- Overuse of computed tomography for minor head injury in young patients: an analysis of promoting factors**  
M Cellina, M Panzeri, C Floridi, CMA Martinenghi... - *La radiologia* ..., 2018 - Springer  
... In our study, only special- ized physicians work in our **emergency room**, and the level of ... in the management of polytrauma patients: indi- cations for MDCT imaging in **emergency** radiology ...  
Melnick ER, Szlezak CM, Bentley SK, Dziura JD, Kotlyar S, Post LA (2012) CT overuse for ...  
☆ 🔍 Related articles All 3 versions

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# Peer-Reviewed Comprehensive Report: the Negatives

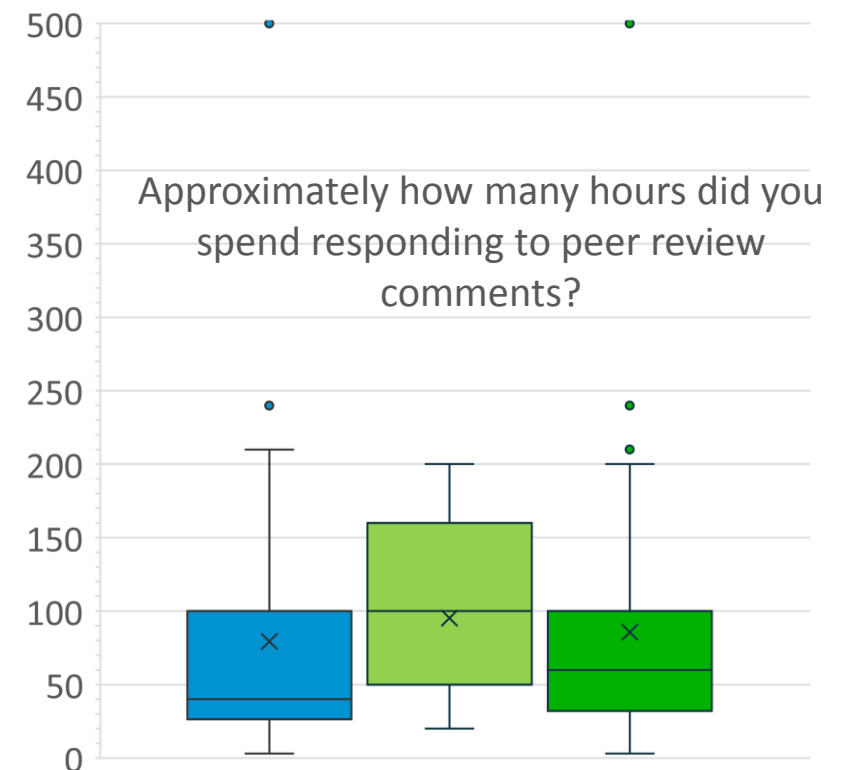
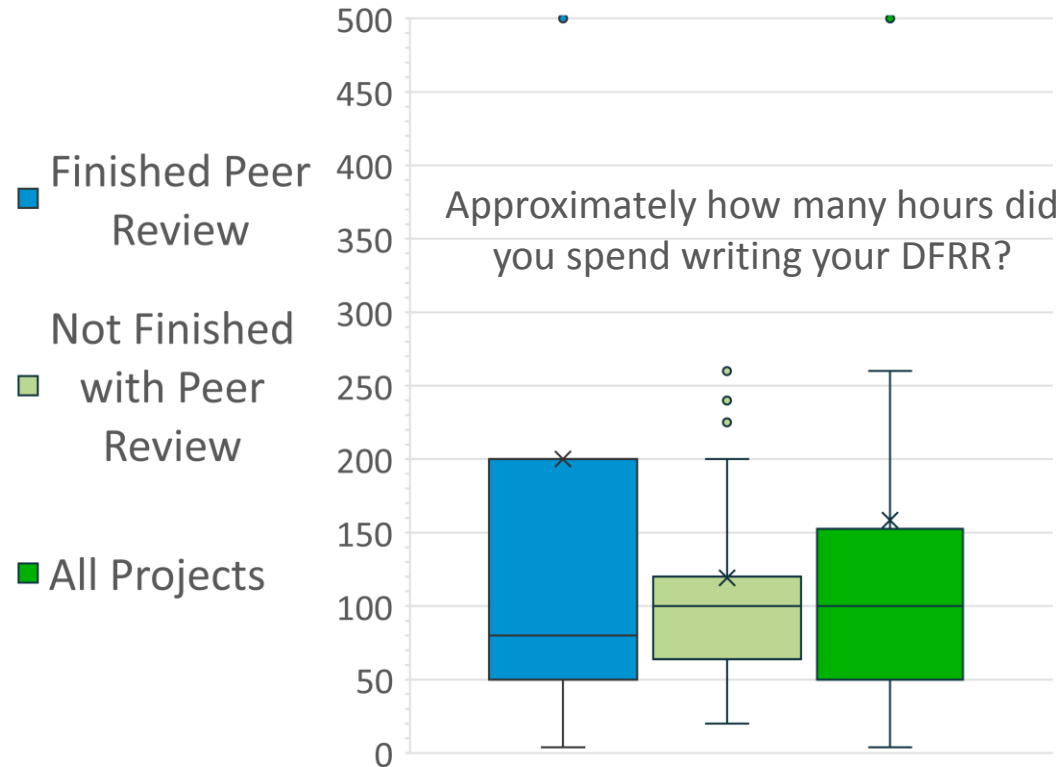


- Many hours for awardees to prepare
- Writing the report competes with writing journal articles.
- Peer review delays release of results to the public.
- Expense of an elaborate external review process

# JAMA Viewpoint Included 2018 PI Survey Results



- Intern project
- 99 PIs (52%) who had entered peer review responded to SurveyMonkey survey.
- PIs reported hours spent writing and revising the DFRR



# Peer-Reviewed Comprehensive Report: the Positives



- Meets the legislative requirement to report all results.
- Transparent public reporting → PCORI reputational considerations

Externalities (speculative):

- Public
- Awardees
- Other researchers and funders
- PCORI

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# Summary: A Model of Trustworthy Comprehensive Reporting of Research



- Governmental mandates have led to a first: a funder that requires a peer-reviewed comprehensive report of all completed research.
- Responding to this mandate requires considerable expense and effort by both awardees and PCORI.
- A comprehensive peer-reviewed research report may benefit many stakeholders (speculation).
- Could transparent, peer reviewed reporting of all research findings be part of the solution to public distrust of the research enterprise

# AHRQ/PCORI LHS K12 Mentored Career Development Program

## Genesis, Overview, and Roadmap

Carly Parry, Senior Advisor, HDDR





# Background and Purpose: K12 Institutional Mentored Career Development Program



- The K12 Institutional Mentored Career Development Program
  - Builds on the work of a Technical Expert Panel, convened by AHRQ (2016) and including PCORI representation to develop a framework and competencies for Learning Health Systems Researchers.
- A summary and report from the TEP appear on AHRQ's website
  - <https://www.ahrq.gov/sites/default/files/wysiwyg/funding/training-grants/lhs-corecompetencies.pdf>
  - <https://www.ahrq.gov/sites/default/files/wysiwyg/funding/training-grants/lhsabstract.pdf>

**Definition of a Learning Health System Researcher:** "An individual who is embedded within a health system and collaborates with its stakeholders to produce novel insights and evidence that can be rapidly implemented to improve the outcomes of individuals and populations and health system performance"

# 7 Domains emerged

- Domain 1: Systems Science
- Domain 2: Research Questions and Standards of Scientific Evidence
- Domain 3: Research Methods
- Domain 4: Informatics
- Domain 5: Ethics of Research and Implementation in Health Systems
- Domain 6: Improvement and Implementation Science
- Domain 7: Engagement, Leadership, and Research Management

# Purpose: K12 Institutional Mentored Career Development Program



- The purpose of the K12 Institutional Mentored Career Development Program is:
  - To train clinical and research scientists to conduct PCOR within learning health systems (LHS) focused on generation, adoption and application of evidence to improve the quality of care and patient outcomes
- The Program incorporates the PCORI Methodology Standards and requires applicants/awardees to address how patient centeredness, patient engagement, health disparities, and health equity will be incorporated in the training plans and ideally operationalized into scholars' research projects.
- The RFA encouraged collaboration with PCORnet sites, seeking to leverage PCORI's significant investment in Clinical Data Research Networks
- This is a unique partnership that has leveraged AHRQs expertise in managing training awards and PCORI's expertise in conducting PCOR and development of learning collaboratives

# Program Objectives



1. Develop and implement a training program including didactic and experiential learning, that embeds scholars at the interface of research, informatics, and clinical operations within LHS.
2. Identify, recruit, and train clinician and research scientists committed to conducting PCOR in health care settings to generate new evidence facilitating rapid implementation to improve quality of care and patient outcomes
3. Establish Centers of Excellence in Learning Health System Research Training focusing on the application and mastery of the newly developed core LHS researcher competencies (see [www.ahrq.gov/LHStrainingcompetencies](http://www.ahrq.gov/LHStrainingcompetencies))
4. Support a learning collaborative across funded Centers of Excellence to promote cross institutional scholar-mentor interactions, cooperation on multi-site projects, dissemination of project findings, methodological advances, and development of a shared curriculum.

# Current Status

- The Funding Opportunity Announcement was released in September 2016: <https://grants.nih.gov/grants/guide/rfa-files/RFA-HS-17-012.html>
- Applications were received January 2018
- AHRQ and PCORI completed complementary reviews
- Awards were made to 11 institutions September 19, 2018, with a start date of September 30, 2018 for all sites
- The learning collaborative was launched and a first Program Director's meeting was held in Rockville in March 2019

# Press Release, AHRQ & PCORI Websites



## AHRQ and PCORI Announce Awards to Support The Next Generation of Learning-Health-System Researchers

Press Release Date: September 11, 2018

The Agency for Healthcare Research and Quality (AHRQ) and the Patient-Centered Outcomes Research Institute (PCORI) today announced \$40 million in grants to support the training of clinician and research scientists to conduct patient-centered outcomes research within learning health systems (LHS).

Organizations that apply for these grants will be required to demonstrate a systematic generation, adoption, and implementation of evidence that ensures that evidence is updated and shared in decision making. Learning health systems are characterized by rich sources of data and a culture of continuous learning.

The new LHS Centers of Excellence will be required to conduct PCOR and implement research scientists will work to address how healthcare organizations can learn from their data and experiences to improve patient outcomes.

PCORI and AHRQ announced that the new LHS Centers of Excellence will be required to conduct PCOR within learning health systems. Research scientists will be required to address how healthcare organizations can learn from their data and experiences to improve patient outcomes.

"Learning health systems aim to ensure that evidence is updated and shared in decision making. Learning health systems are characterized by rich sources of data and a culture of continuous learning."

"The researchers supported by these grants will be required to demonstrate a systematic generation, adoption, and implementation of evidence that ensures that evidence is updated and shared in decision making. Learning health systems are characterized by rich sources of data and a culture of continuous learning."

### Project Summary

#### Awarded Under the AHRQ-PCORI Institutional Mentored Career Development Program (K12)

In September 2018, PCORI and the Agency for Healthcare Research and Quality (AHRQ) [announced awards](#) totaling \$40 million over five years to 11 institutions to establish Learning Health Systems (LHS) Centers of Excellence, listed below, which utilizes the joint efforts of clinician and research scientists to improve patient outcomes.

The initiative is focused on the generation, adoption, and implementation of evidence to improve patient outcomes. The initiative is focused on the generation, adoption, and implementation of evidence to improve patient outcomes. The initiative is focused on the generation, adoption, and implementation of evidence to improve patient outcomes.

A [Funding Opportunity Announcement](#) (FOA) was released on September 11, 2018, with an application deadline of October 15, 2018.

The institutions funded under this initiative will produce the next cadre of LHS researchers who have the skill sets to conduct, apply, and implement patient-centered outcomes research to improve quality of care and patient outcomes in a learning health system. Please click on the grant number for more information about each program:

## Supporting the Next Generation of Learning Health Systems Researchers

### Grants Awarded Under the AHRQ-PCORI Institutional Mentored Career Development Program (K12)

The Agency for Healthcare Research and Quality (AHRQ) and the Patient-Centered Outcomes Research Institute (PCORI) have awarded \$40 million in grants over 5 years to 11 institutions to support the training of clinician and research scientists to conduct patient-centered outcomes research within learning health systems (LHS).

The institutions funded under this initiative will produce the next cadre of LHS researchers who have the skill sets to conduct, apply, and implement patient-centered outcomes research to improve quality of care and patient outcomes in a learning health system. Please click on the grant number for more information about each program:



# Program Details

- The awards support
  - 11 institutions (Centers of Excellence)
  - Up to 5 years per institution
  - ~\$800,000/year in total annual costs per project
  - 40 scholars will be appointed in Year One, with an estimated 92 scholars appointed over the 5-year program
  - Scholar appointments range from 2-3 years



# Awards

Grant #	Institution	Program
HS026396	Albert Einstein College of Medicine	The Center of Excellence in Promoting LHS Operations and Research at Einstein/Montefiore (EXPLORE)
HS026393	Children's Hospital of Philadelphia	PEDSnet Scholars: A Training Program for Pediatric Learning Health System Researchers
HS026390	Indiana University School of Medicine	Leveraging Infrastructure to Train Investigators in Patient-Centered Outcomes Research in the Learning Health System (LITI- PCORLHS)
HS026369	Kaiser Permanente Washington Health Research Institute	CATALyST: Consortium for Applied Training to Advance the Learning health system with Scholars/Trainees
HS026385	Northwestern University	A Chicago Center of Excellence in Learning Health Systems Research Training (ACCELERAT)
HS026370	Oregon Health and Science University	NW Center of Excellence & K12 in Patient-Centered Learning Health Systems Science
HS026407	University of California, Los Angeles	Stakeholder-Partnered Implementation Research and Innovation Translation (SPIRIT) program
HS026383	University of California, San Francisco	UCSF Learning Health System K12 Career Development Program
HS026379	University of Minnesota	Minnesota Learning Health System Mentored Career Development Program (MN-LHS)
HS026372	University of Pennsylvania	Learning Health Systems Mentored Career Development Program
HS026395	Vanderbilt University Medical Center	Learning Health System Scholar Program at Vanderbilt



# Learning Collaborative Goals

- To serve as a forum to promote cross institutional scholar-mentor interactions, collaboration on projects, dissemination of project findings and methodological advances, and the development of shared curriculum.
- To provide a platform for participants to share their experiences to accelerate learning and implementation of best practices along with participating in trainings.
- To develop an online shared curriculum of training LHS researchers that can serve as a comprehensive and efficient training model and expand reach of the program to other health systems.

# Learning Collaborative Activities

Progress toward achieving goals is facilitated via:

- Active participation in monthly web-ex calls
- Utilizing the learning collaborative SharePoint site
- Attending in-person annual meetings

## Training Resources

- PCORI
- AHRQ

# Learning Collaborative Roles



- AHRQ leads and provides support for the learning collaborative.
- AHRQ works closely with PCORI to provide PCOR-specific training opportunities.
- PCORI and AHRQ evaluate progress reports, collaborate on training materials and PD meeting materials/learning
- All LHS K12 Program Directors are required to participate in the learning collaborative.
- PCORI team includes Science and Engagement

# Key Milestones

- A shared curriculum to train the next cadre of LHS researchers to conduct PCOR:
  - July-September 2020: Synthesis of best practices
  - March 2021: Posting guidelines of shared online curriculum
  - March 2021: Online delivery recommendations
  - June 2021: Dissemination Plan
- A program evaluation report:
  - March 2019: Common evaluation metrics
  - September 2019: Data Collection tool ready
  - October 2020: Yearly data collection

# Related Activities

- March 18, 2019 LHS Program Director's meeting
- February 19-21 Embedded Research Conference

# March 18, 2019 Program Director's Meeting



- Agenda
  - Common evaluation metrics discussion
  - Building a shared curriculum
  - Engagement training resources
  - PCORI Methodology standards
  - Mentor and System Relationships
  - Administrative housekeeping

# Embedded Research Conference:

- “Accelerating the Development of Learning Healthcare Systems through Embedded Research” expert meeting
- February 19-21
- Funded by PCORI, AHRQ, VA HSR&D
- 100+ attended, hosted by Kaiser Permanente Southern California & Academy Health
- Embedded research operationalized as: research conducted through intensive collaborations between researchers and policy/practice stakeholders
- (Summary report pending)

# Purpose & Objectives

- Purpose: the meeting was Intended to facilitative growth and improvement of health system-based "embedded" research programs ( a core element of the learning healthcare system)
- Objectives:
  - 1) identify and examine a range of org. models and governance structures for embedded research
  - 2) Identify the types of questions, methods and designs that best balance practice and science considerations
  - 3) Disseminate specific recommendations and guidance for establishing and managing and embedded research program (report that outlines workgroup deliberations & recommendations, plans for dissemination, follow-up)



# **BREAK**

---

We will return at approximately 2:30pm  
EDT

# PCORI RESEARCH PORTFOLIO DATA MINING TO INFORM THE PRACTICE OF ENGAGEMENT IN RESEARCH

---

*A Qualitative Study to Understand the Influence and  
Effects of Engagement in PCORI-Funded Studies*

**Maureen Maurer**

Principal Researcher, AIR

# Team members



## AIR Team

- Maureen Maurer, Project Director
- Rikki Mangrum, Deputy Project Director
- Tandra Hilliard, Task 1 Lead
- Jessica Arnold, Project Manager
- Andrew Amolegbe
- Kirsten Firminger
- Karen Frazier
- Tamika Cowans
- Charis Yousefian
- Marla Clayman
- Tom Workman
- Emily Elstad

## PCORI Team

- Kristin L Carman
- Rachel Mosbacher
- Andrea Heckert
- Julie Kennedy Lesch
- Laura Forsythe
- Krista Woodward
- Beth Nguyen

PCORI's Patient Engagement Advisory Panel provided input prior to interviews and during analysis

# Our Time Today



- Project purpose and methods
- Findings from interviews with principal investigators (PIs) and partners about the influence and effects of engagement
- Your input on these findings
- Next steps

# Purpose and Methods

---

Research Questions

Sampling Description

Data Collection and Analysis  
Approach

# Project Rationale and Purpose



## Rationale

- We need better, stronger, more robust evidence about the value of engagement in research.

## Purpose

- If patient and stakeholder engagement changes the course of PCORI-funded studies, how did that happen?

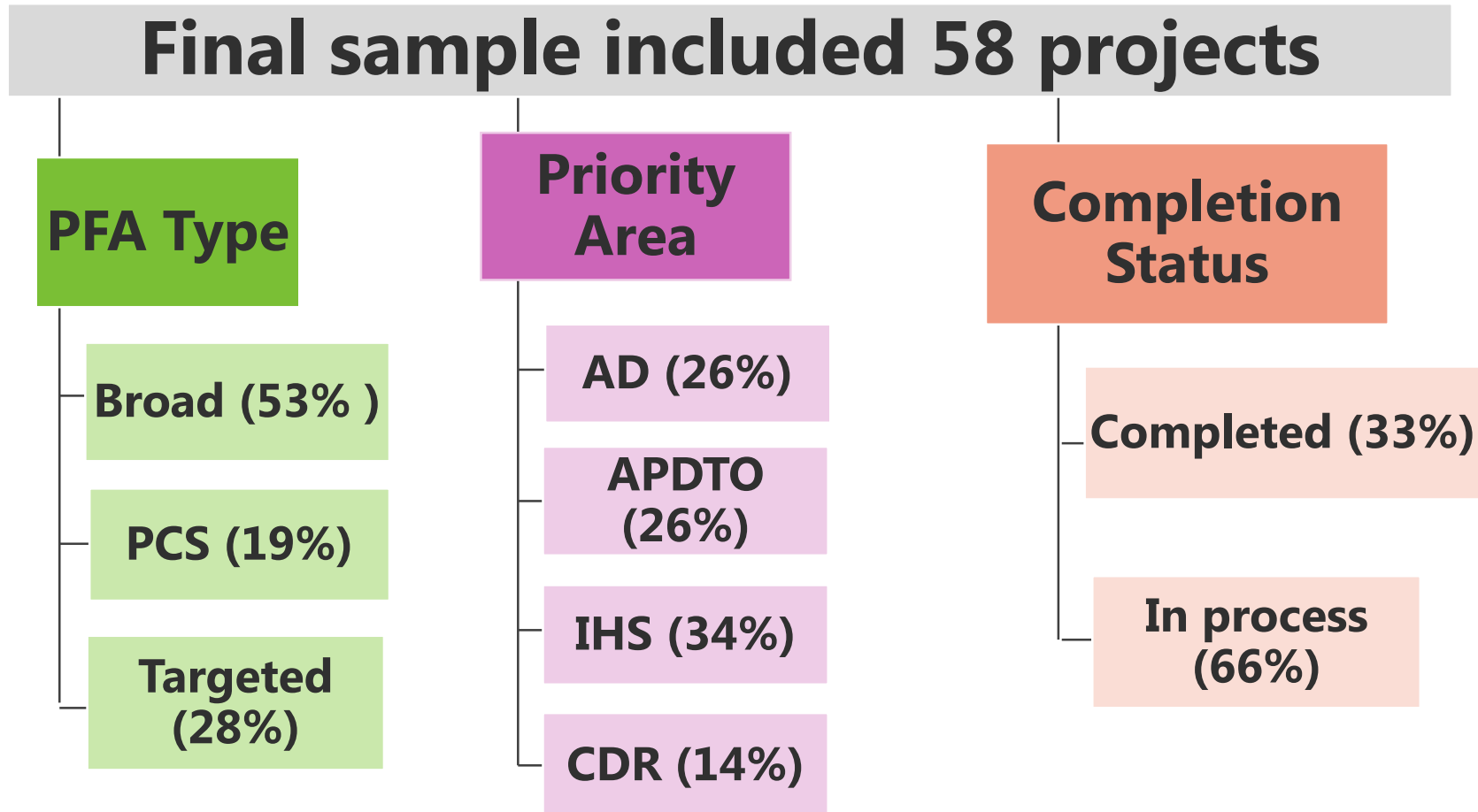
## Research Questions

- How has engagement influenced the planning and conduct of PCORI-funded studies and the dissemination of their results?
- What changes to the study resulted from that influence?
- How did PIs and partners perceive engagement as successful or unsuccessful?

# Description of Interview Participants

- We used a **purposeful sampling approach** and selected projects within three primary strata:
  - PFA type
  - Funding priority area
  - Completion status
- In total, we conducted **109 interviews** with **58 PIs** and **51 stakeholders** from **58 projects**.
  - **Roughly half** of the PI interviews included another member of the research team
  - The **majority** of partners interviewed identified as patients

# Description of Final Projects in Sample



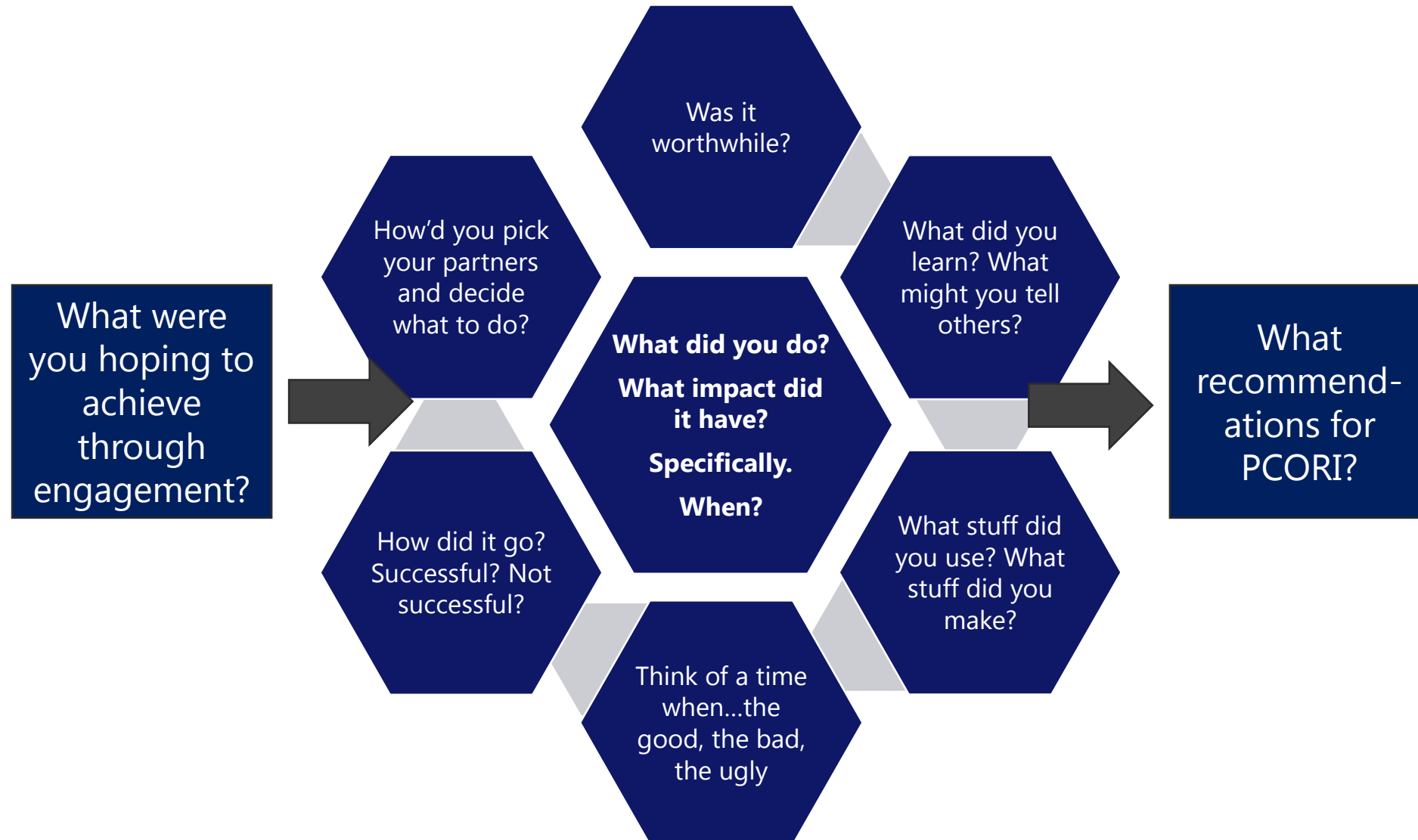


# Sample Rationale

The purpose of this stratification or segmentation approach was to help achieve a diversity of projects within the sample.

- This study is **not** designed to be **statistically representative or generalizable**.
- It is **not** an **assessment or formal evaluation** of engagement that compares or contrasts performance across PCORI's entire research portfolio.
- However, it **will** result in findings that are **informationally representative with potential transferability**.

# Visual of Protocol



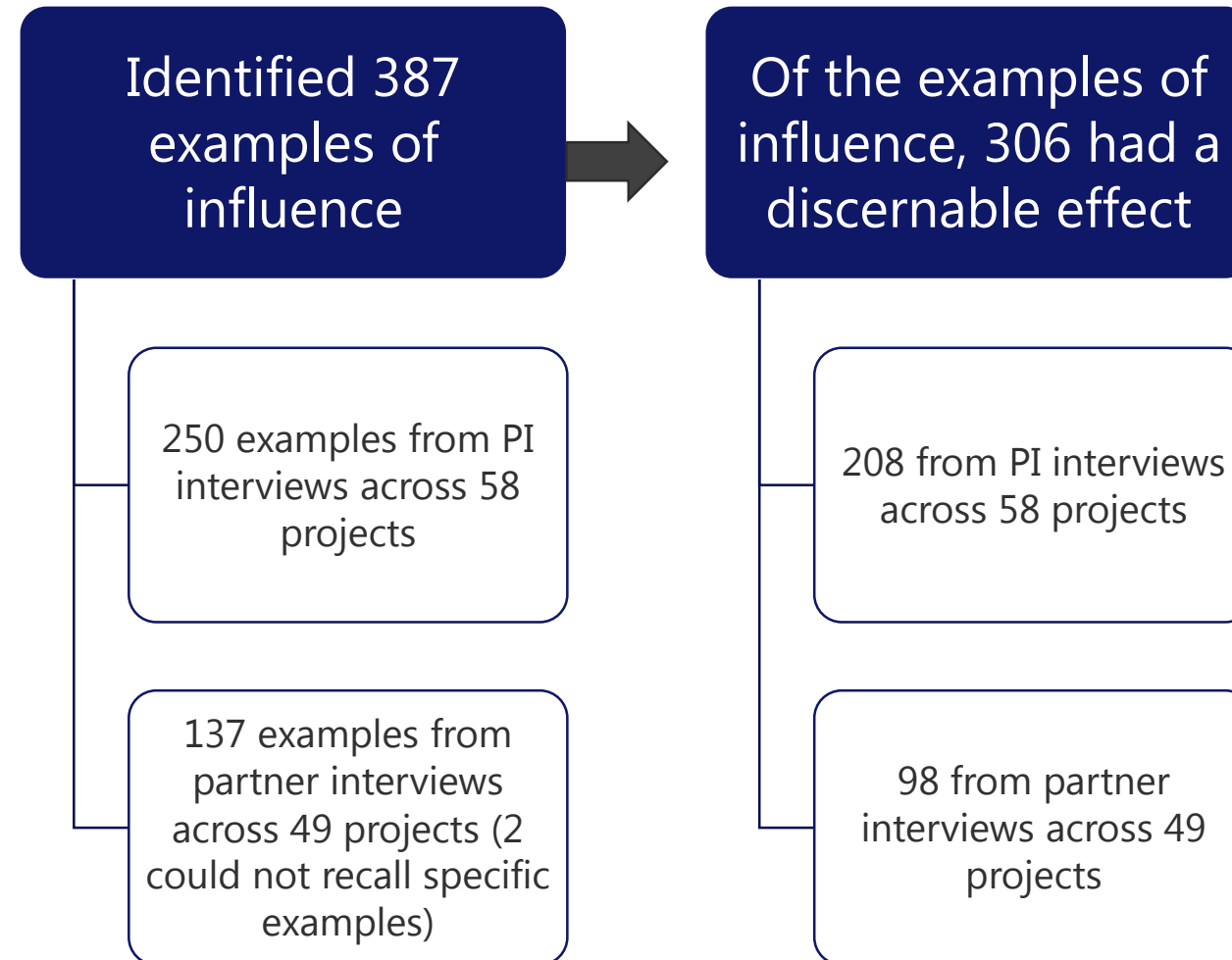
# Findings

---

Describing Influence and Effects of  
Engagement on Studies



# Catalogued Examples of Influence and Effect



# Let's Start with Influence

Exploring the Phenomenon of Influence  
By Examining Specific Examples and  
Stories

# Influence Was Dynamic and Had Multiple Components

## **PIs and partners described engagement as**

- Dynamic, taking different forms at different times.
- An integral part of the study rather than an external influence

## **Each example influence reflected multiple components that met the needs the activity or task**

- Who was involved
- When the influence occurred
- What they influenced
- HOW stakeholders exerted influence = main focus of this analysis

# Components of Influence: What We Learned about Who, When, and What

## Who

- Different stakeholders could be involved at different times
- Relationships between researchers and diverse stakeholders perceived as foundation of successful engagement

## When

- Occurred in all phases of the study, including engagement process itself
- Perceived as working best when stakeholders were involved early and often

## What – too many examples to present in a brief presentation, but

- *Study conceptualization and execution*: identify research questions, choose study design, broaden or narrow inclusion criteria, participate or lead efforts to recruit, enroll, and retain participants, conduct data collection; interpret results
- *Study materials and products*: help develop intervention materials, revise recruitment and informed consent materials, develop dissemination products
- *Engagement process*: designing or redesigning engagement process, recruit new partners
- Take a closer look at recruitment and enrollment of study participants...

# Examples of Influence: Recruitment and Enrollment

- Partners conducted outreach activities to sites and patients, such as
  - Leveraging contacts at potential sites
  - Conducting meet-and-greets at clinics to recruit patients
  - Holding or attending community events to interest potential patients
  - Distributing information about the study through their own communication channels
  - Allowing researchers to leverage their organization's name and reputation to help promote the study
- Partners influenced recruitment materials, such as
  - Creating flyers, letters, and informed consent materials
  - Advising and cautioning researchers how content, messaging, or language might be misinterpreted during recruitment
  - Simplifying materials, making them shorter and clearer, and improving the language and cultural appropriateness of the content.
- Partners enrolled patients in the study



# Recruitment and Enrollment: Quotes

*"They were having trouble finding participants. We made several suggestions on other recruitment avenues. We said to go to doctor's offices, mail them, different things. Contact patients rather than waiting for them to come in for appointments. And they did that and that, they said, 'thank you so much for those suggestions because we employed them and now we've recruited who we've needed to because of those suggestions.'" (Partner)*

"We started having issues with enrollment and tried to enroll more people. We met enrollment, actually exceeded enrollment goals. But we'd have to credit our stakeholders in figuring out different strategies for each. In my other studies, I've changed criteria. We considered that. Should we loosen up the criteria?... We stuck to our guns and didn't liberalize. And at the end, shared strategies of sites, building relationships with community-based organizations in part through stakeholders." (PI)

# Examining How Stakeholders Exerted Influence: Classifying Examples into Five Types of Influence



**Co-producing:** Stakeholders and researchers work together or collaborate on study design and execution, including co-conceptualizing study design, co-executing study tasks or partner-led task.



**Re-directing:** Stakeholders shift the direction of or create new plans, approaches, materials, or processes.



**Refining:** Stakeholders edit or modify existing plans, approaches, materials, or processes.

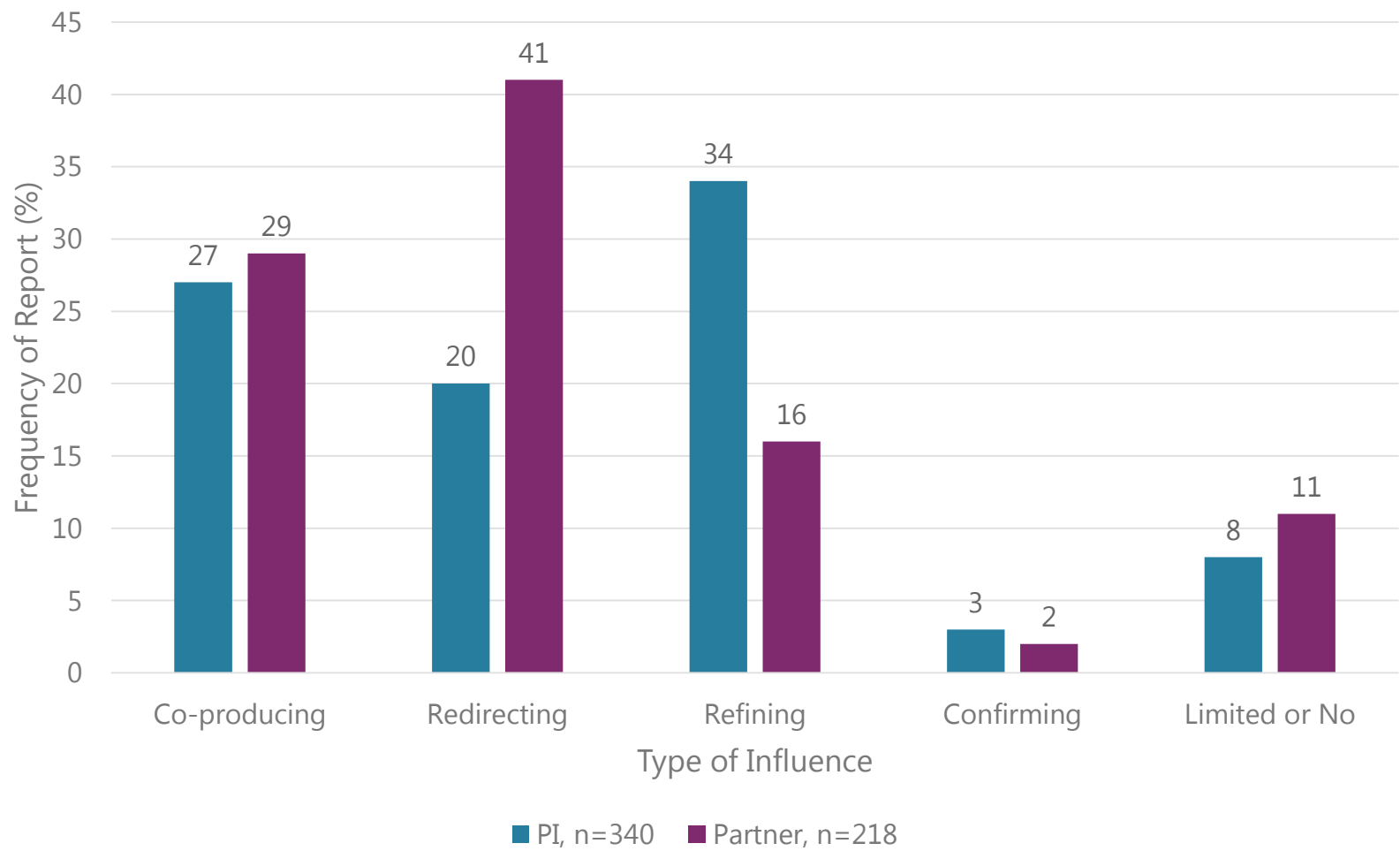


**Confirming:** Stakeholders review, confirm, or validate existing plans, approaches, materials, or processes.

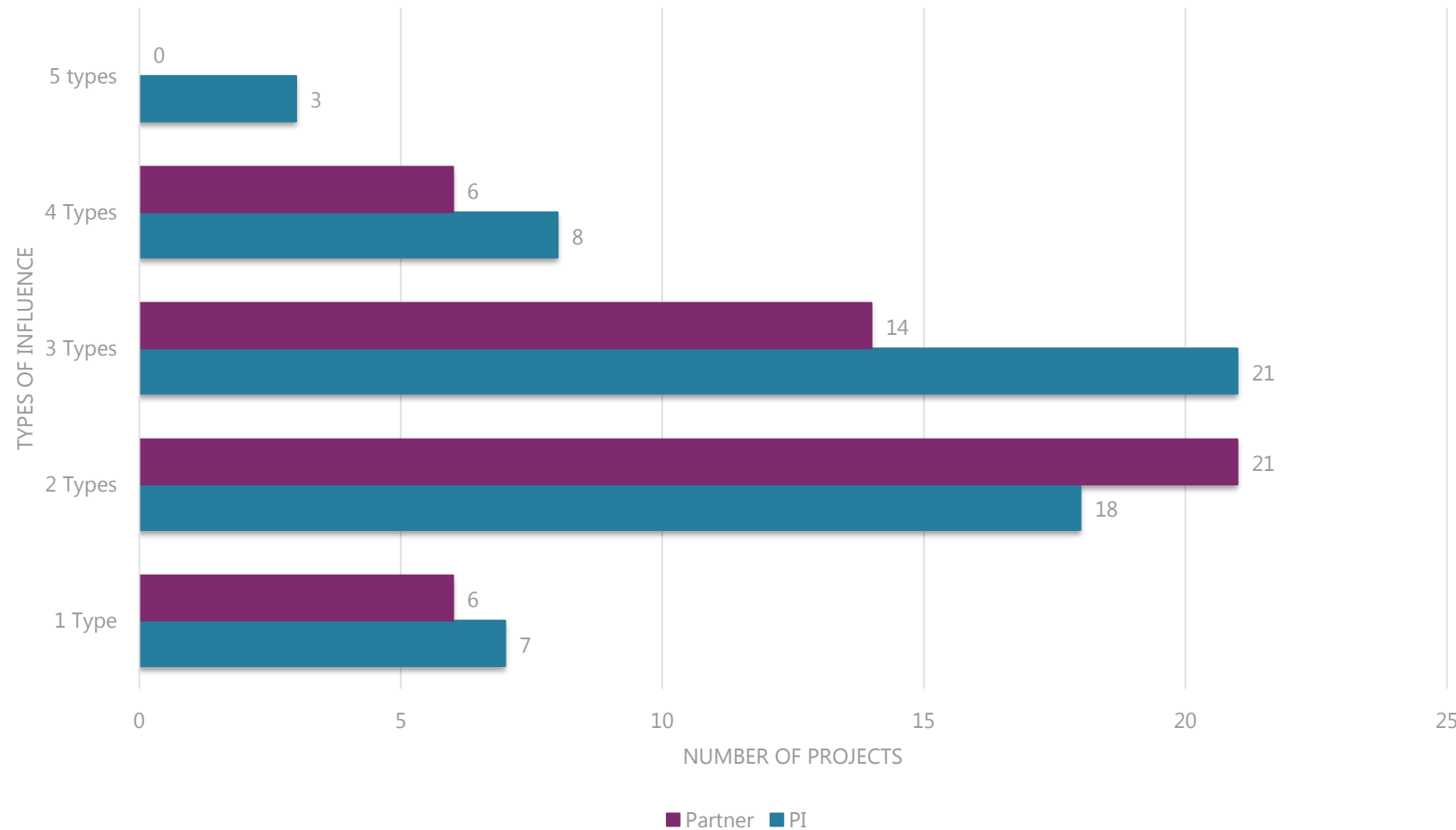


**Limited or no influence:** Stakeholders may have limited or no influence because suggestions could not be implemented (by PCORI, PI, IRB, or stakeholder organization), or stakeholders did not have much input to offer

# Frequency of Types of Influence



# Most Projects Exhibited Multiple Types of Influence



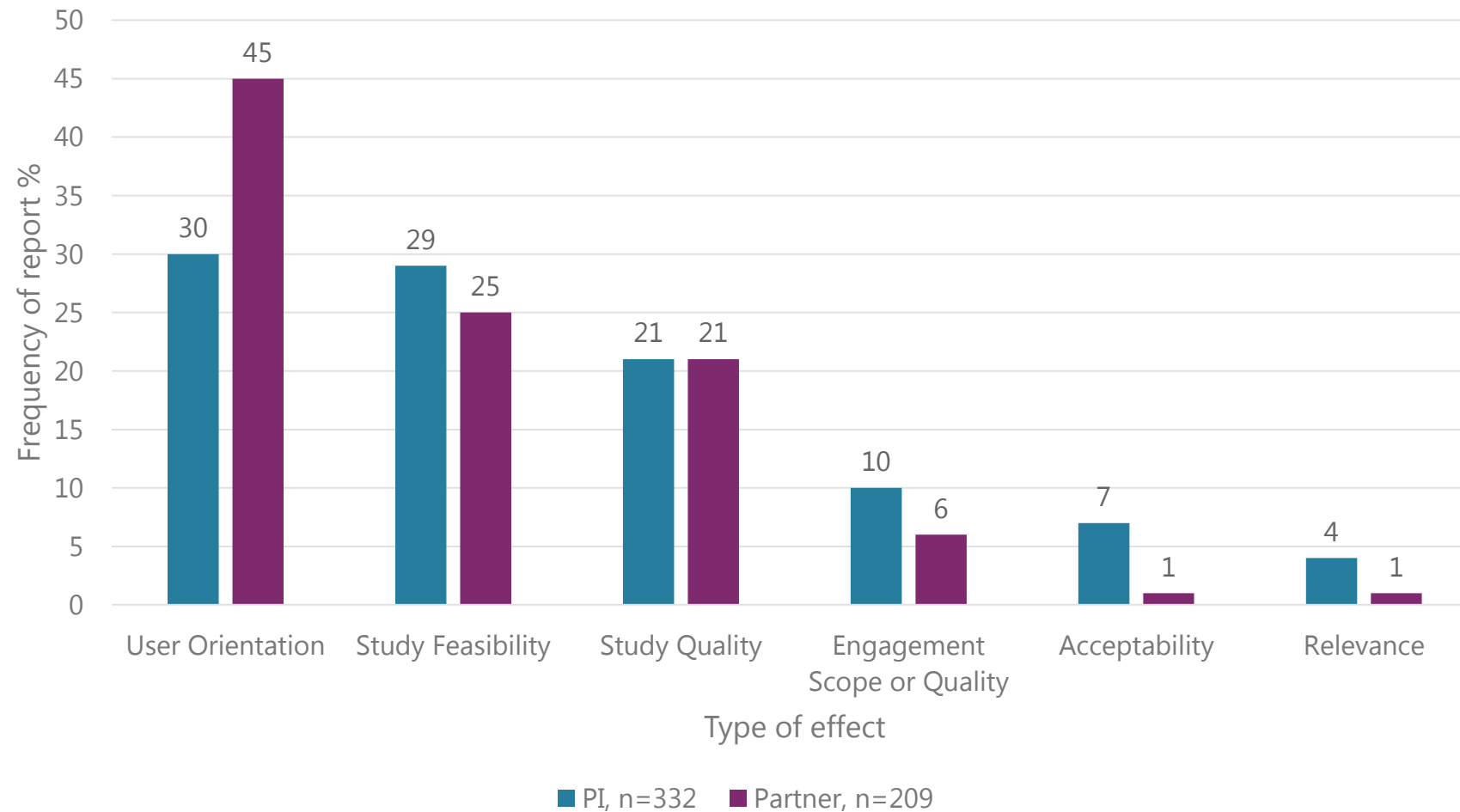
# Let's Look at the Effects

How Did PIs and Partners Report How Engagement Made a Difference?

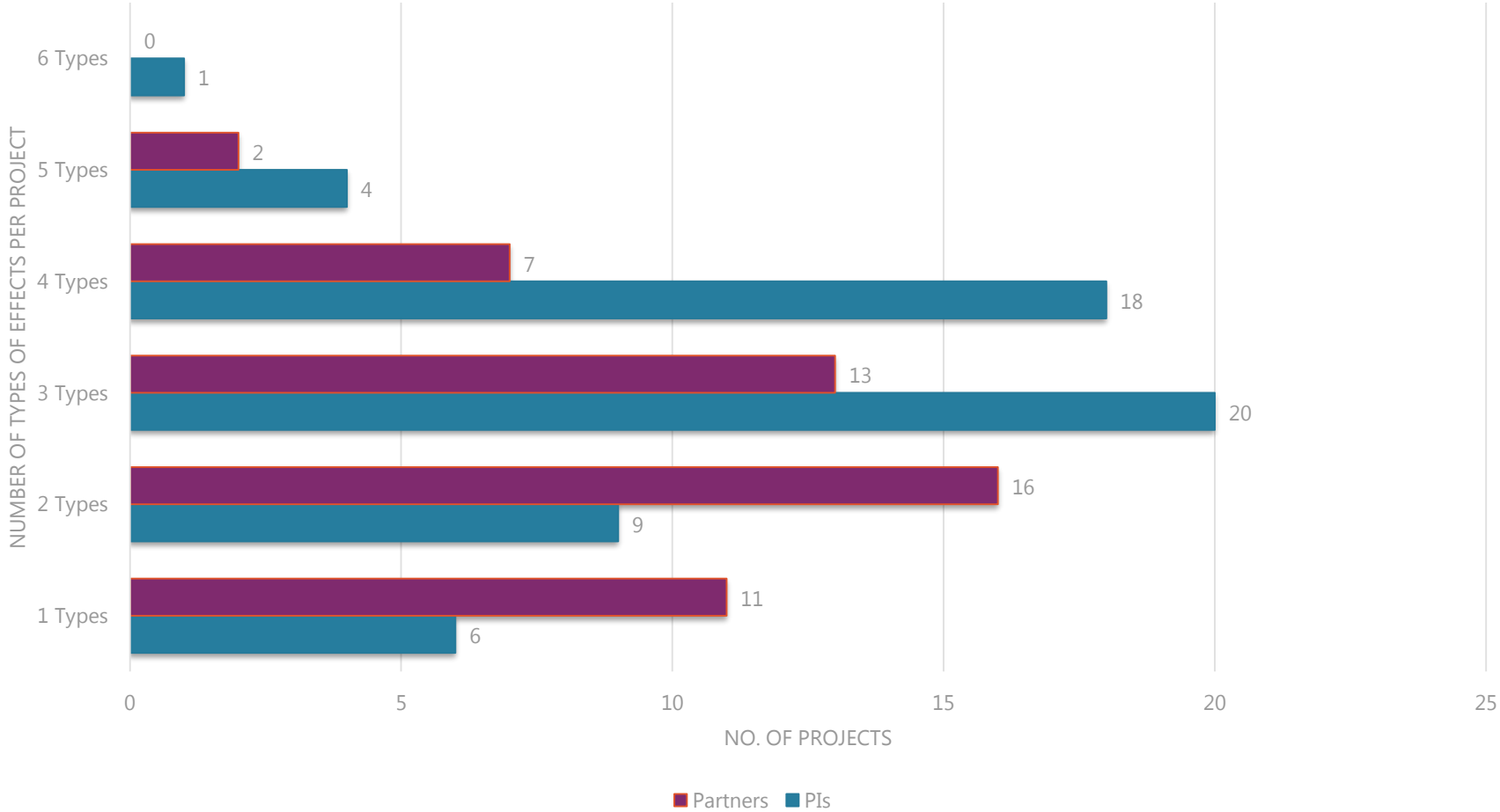
# Types of Effects

Effect	Description
<b>User orientation</b>	Study reflects perspectives, needs, and preferences of patients, providers, or other stakeholders
<b>Study feasibility and execution</b>	Study can be executed as planned, the planned methods will work to recruit sites or patients, deliver the intervention, and collect needed data
<b>Study quality</b>	Study is comprehensive, robust, and rigorous, including quality of study materials
<b>Engagement scope or quality</b>	Study represents perspectives of all stakeholders and partners have information and support to do work
<b>Acceptability</b>	Study is one that people will be willing to participate
<b>Relevance</b>	Study will product results people can use

# Frequency of Types of Effects Reported by PIs and Partners



# PIs and Partners Reported Multiple Types of Effect in Each Project





# Examining How Stakeholders Exerted Influence: Identifying Critical Incidents



- A **critical incident** can be described as an observable, specific behavior that exemplified—either positively or negatively—how engagement influenced the study.
- Each critical incident captures:
  - Context—what led up to the situation?
  - What happened—what did the researchers and partners do, what were their behaviors?
  - What was the result—what was the nature of the influence or change?
  - What was the effect—what impact did the influence or change have on the study, engagement, person, or organization?
- We identified **300 critical incidents** across 57 projects

# Examples of Critical Incident

- Example 1: Researchers were experiencing low survey completion. Partners volunteered to help and insisted that online-only wasn't the best mode of survey administration for people living in rural areas or from vulnerable populations. Researchers added a phone survey mechanism to improve user orientation. Ultimately, over half (50-60% as reported by PI) of participants preferred to complete the survey via phone, which surprised the researchers.
- Example 2: The target stakeholder clinic and target population were wary of research in general and of the original intervention specifically, so the PI undertook six months of pre-engagement and leveraged research team members who were from the target community; these strategies continue into the study. The clinic, and eventually the patient stakeholders, taught the research team a great deal about what the clinic can do and the community needs. They made substantial changes to the intervention itself, to make it less burdensome and costly, and more appealing. These changes included shortening it and changing the format, as well as making attendance at group sessions more flexible. The original intervention was unworkable. Revisions made it feasible for the clinic to offer it and for patients to participate.

# Identified Five Pathways of Influence and Effect from the 300 Critical Incidents

<b>Mapping out together or collaborating</b>	Researchers and partners worked together to design and develop (“map out”) components in the study. Collaboration happened through a single engagement event with a big impact (e.g., a summit meeting) or continual collaboration.
<b>Solving problems</b>	Researchers and partners convened to address engagement- or study-related challenges (PI- or partner-initiated), often related to recruitment and retention and survey completion.
<b>Negotiating access to clinics or communities to do the study</b>	Researchers and partners negotiated access to or developed external relationships to achieve buy-in and participation from needed organizations or communities.
<b>PI or researchers initiate request for partner review and response</b>	Researchers asked partners to do a specific activity, requested input on specific issues, or asked for advice or perspectives. Partners were in a reactive role in which they responded to what researchers presented.
<b>Partner initiates or insists on opportunity to engage</b>	Partners requested inclusion in study activities and decisions, brought up new ideas, identified potential problems or initiated materials, plans, or ideas. PIs were in a reactive role, responding to partners’ requests.

# Examples of Critical Incident

- Example 1: Researchers were experiencing low survey completion. Partners volunteered to help and insisted that online-only wasn't the best mode of survey administration for people living in rural areas or from vulnerable populations. Researchers added a phone survey mechanism to improve user orientation. Ultimately, over half (50-60% as reported by PI) of participants preferred to complete the survey via phone, which surprised the researchers. → **PROBLEM SOLVING**
- Example 2: The target stakeholder clinic and target population were wary of research in general and of the original intervention specifically, so the PI undertook six months of pre-engagement and leveraged research team members who were from the target community; these strategies continue into the study. The clinic, and eventually the patient stakeholders, taught the research team a great deal about what the clinic can do and the community needs. They made substantial changes to the intervention itself, to make it less burdensome and costly, and more appealing. These changes included shortening it and changing the format, as well as making attendance at group sessions more flexible. The original intervention was unworkable. Revisions made it feasible for the clinic to offer it and for patients to participate. → **MAPPING OUT TOGETHER**

# Examining How Stakeholders Exerted Influence: Other Themes



**PIs or partners reported that stakeholders taught researchers real-world, end-user knowledge or perspectives in 46 projects**

- Teaching produced an effect, even though stakeholders did not exert direct influence on a specific aspect of study design or conduct

**PIs discussed negotiating tensions between engagement input and science**

- Often related to study rigor or design, PIs discussed the need to negotiate input with science best practices
- Sometimes, stakeholder input was not integrated

**PIs and partners described PCORI as an involved stakeholder that can affect the extent of engagement's influence**

- Facilitates = requires; funds; provides structure, resources, and accountability
- Hinders = inflexible contractual mechanisms

# Overall, PIs and Partners Said Engagement Was Worthwhile

*"At first we viewed it a little bit as burdensome, but over time we really started to see the value in it and see the way that it was actually impacting the decisions we were making and how we were carrying out the study so that it would be more relevant to patients and providers on the front lines." (PI)*

*"PCORI and this study has opened my eyes personally to how important my input is. I did not know that until I got involved with this study, how important my voice, how important a patient's voice is in studies. (Partner)*

# Take-Aways

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What questions has this analysis raised?  
What have we learned?

Need your input!



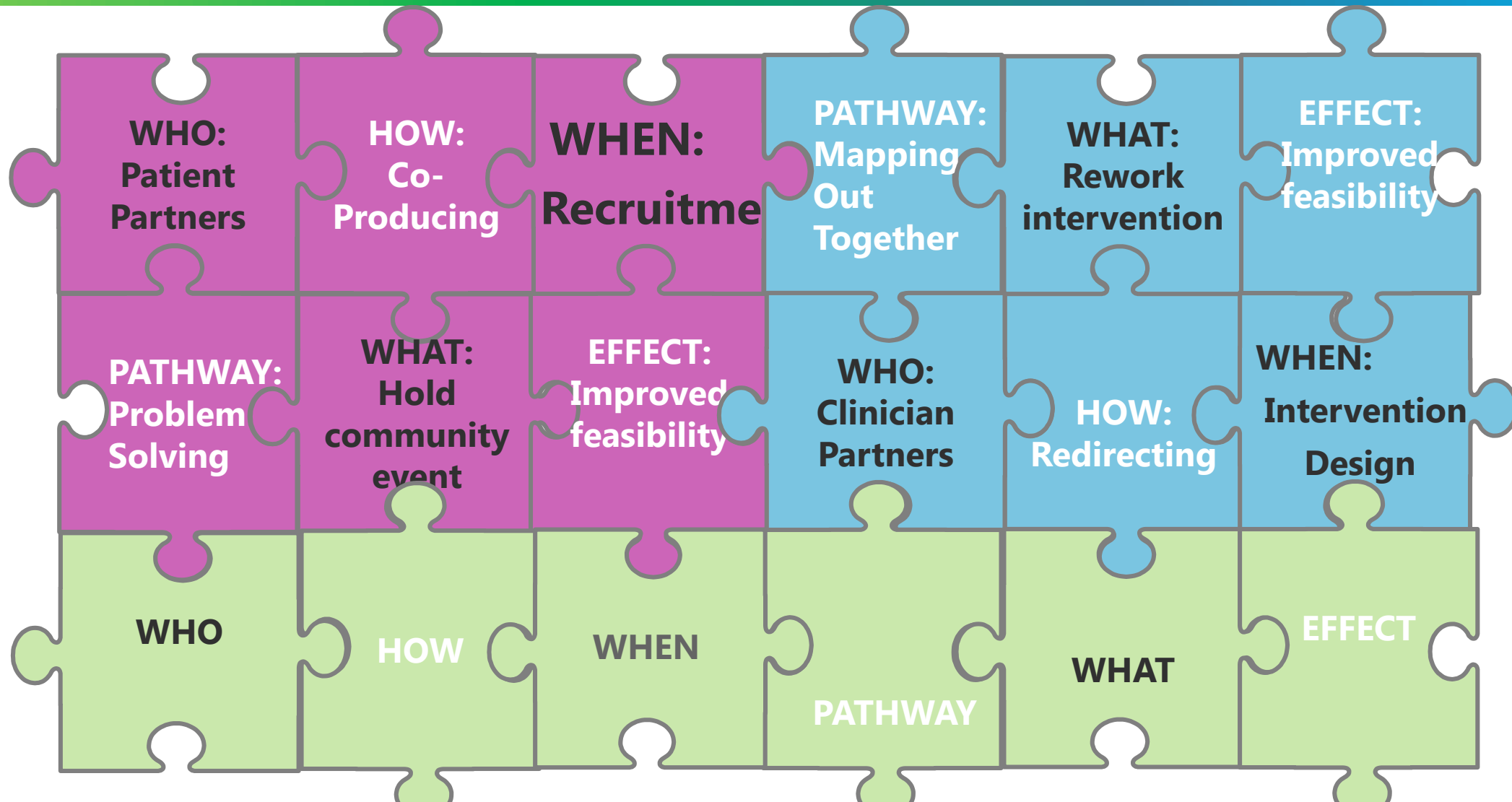
# Our Analysis Raises Additional Questions

- What are the relationships between types of influence and effects and the pathways that connect them?
- What can be said about the influences, effects, and their pathways at the project level?
- What are ways to support research teams when there are tensions between research practices and engagement input, or where there are tensions between stakeholder groups?
- For the in process projects, what are the ultimate effects of engagement?



# What Can We Conclude?

Engagement is complicated, with different components mixing and matching to meet specific project goals and needs

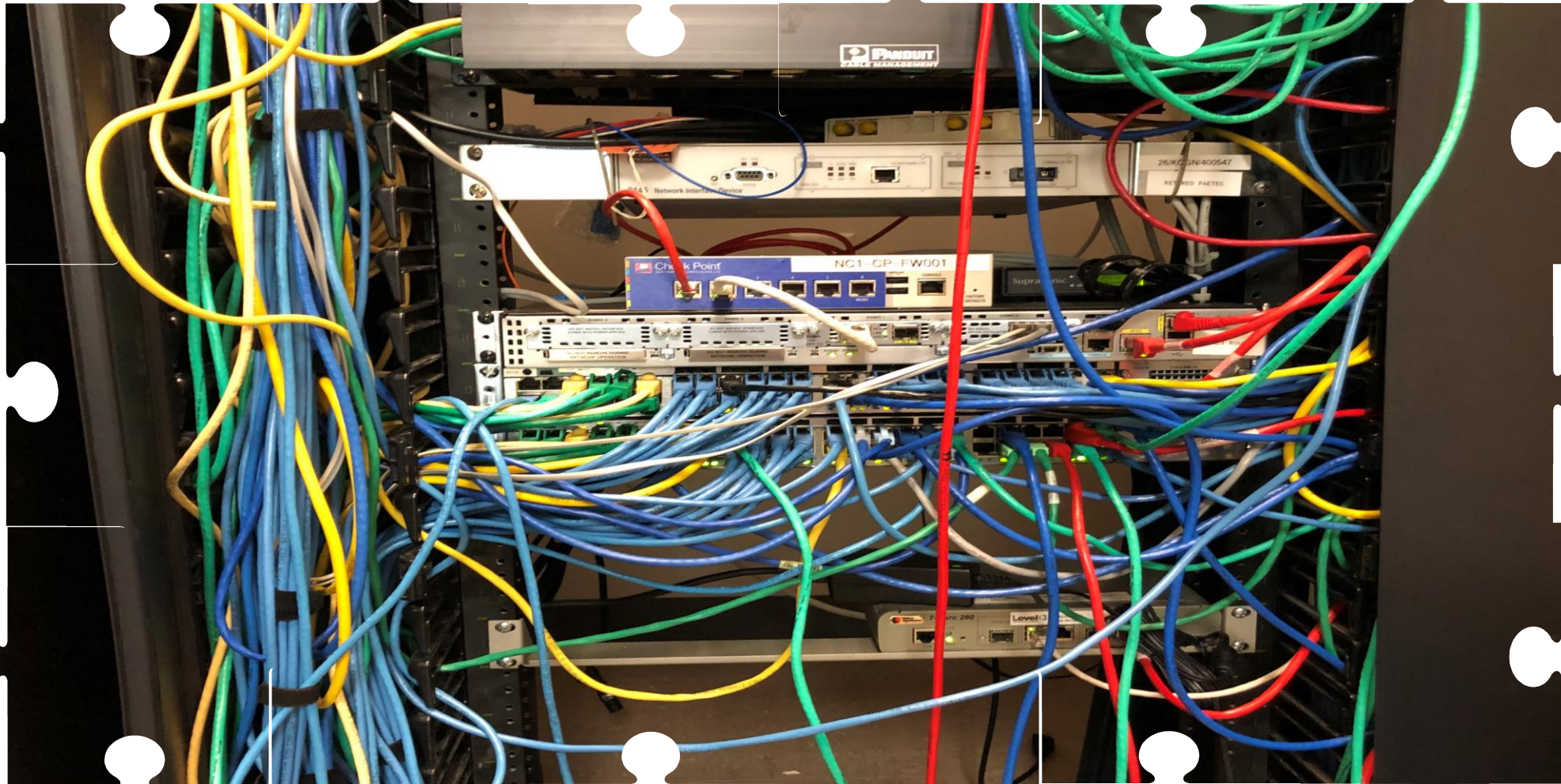


# What Can We Conclude?



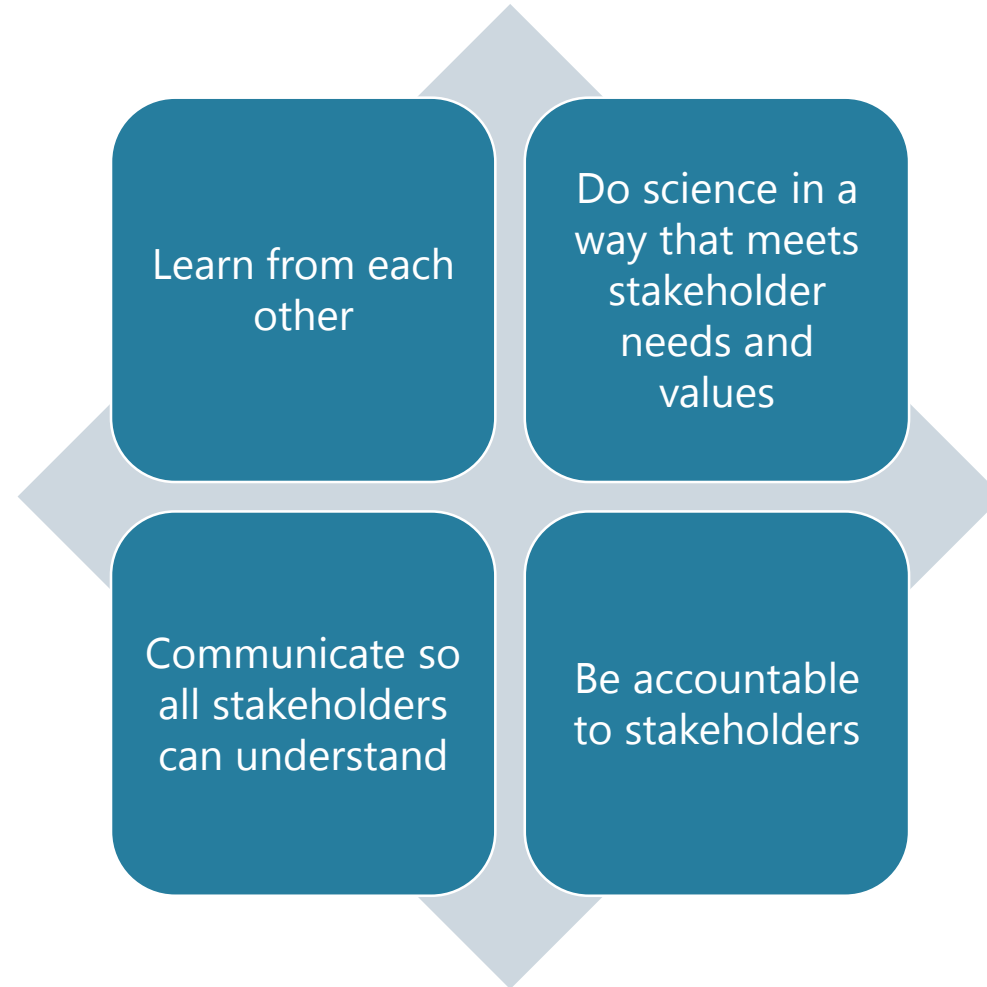


# What Can We Conclude?



# What Can We Conclude?

## Core Outcomes of Engagement Speak to How Research is Being Done Differently



## **Your thoughts and reactions – small and large group discussion in 3 stages:**

1. WHAT: What findings stood out?
2. SO WHAT: Why are those findings important?
3. NOW WHAT: What actions should PCORI take in terms of
  - Presenting findings
  - Further research
  - Developing tools and resources

# Next Steps

- Incorporate your input and finalize report
- Share report with interviewees
- Prepare a manuscript

# Thank You!

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**Maureen Maurer**  
Principal Researcher, AIR  
[mmaurer@air.org](mailto:mmaurer@air.org)



# Closing Remarks

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**ADJOURN**





# Dinner

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**6 PM @ Washington Marriott Georgetown**

1221 22nd St NW, Washington, DC 20037

(DAY TWO - Start time 8:45A)