

Board of Governors Meeting

In-Person and via
Teleconference/Webinar

December 9, 2019
9:00 AM – 1:00 PM ET

Welcome and Introductions

Christine Goertz, DC, PhD

Chairperson, Board of Governors

Josephine Briggs, MD

Interim Executive Director & Acting Chief Science Officer

Agenda



9:00 AM

Call to Order and Welcome

9:00 – 9:15

Consider for Approval: November 19, 2019 Board Minutes

9:15 – 10:15

Executive Director's Report & FY2019 Dashboard

10:15 – 11:15

Patient-Centeredness and Engagement: What Have We've Learned?

11:30 – 12:30

Research Portfolio Exploration Series: Focus on Cancer

12:30 – 1:00

Public Comment Period

1:00 PM

Wrap up and Adjournment

Board Vote

Call for a Motion to:

- **Approve** the Minutes of the November 19, 2019 Board Meeting

Call for the Motion to be Seconded:

- **Second** the Motion
- If further discussion, may propose an **Amendment** to the Motion or an **Alternative** Motion

Voice Vote:

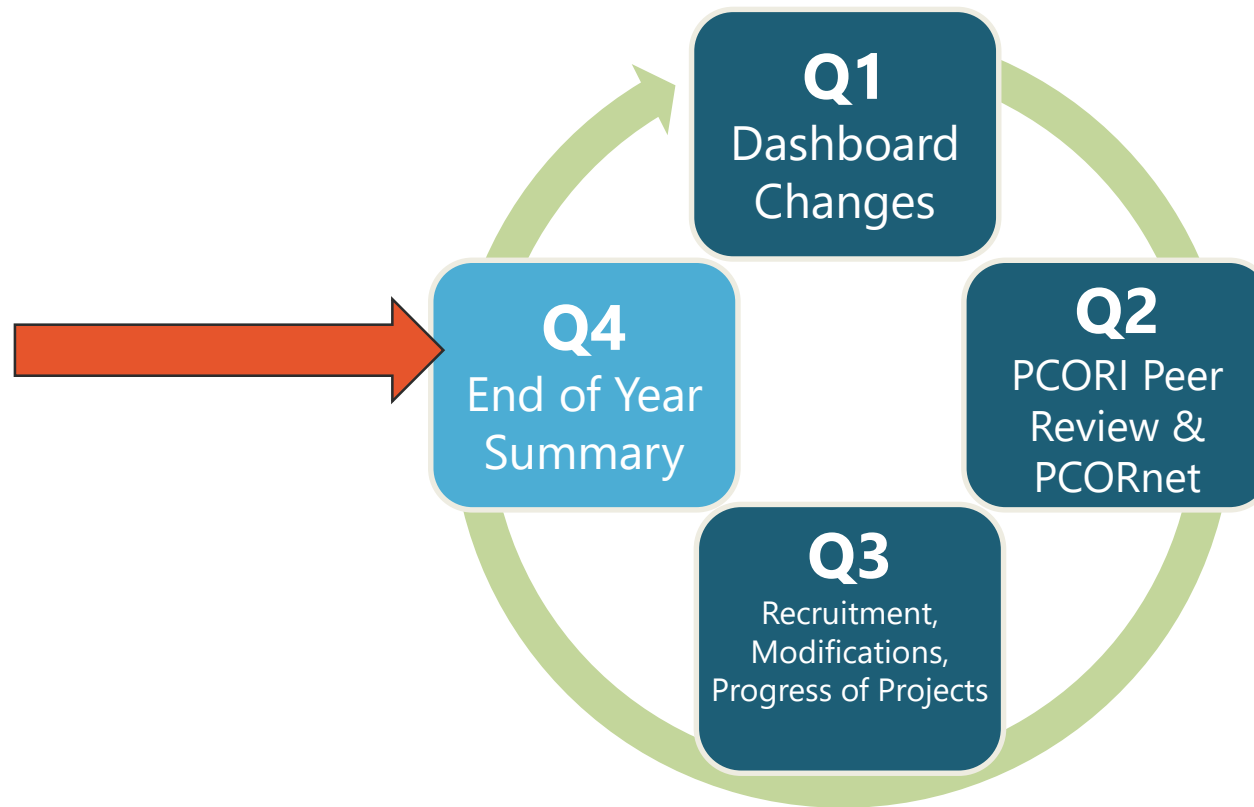
- Vote to **Approve** the **Final** Motion
- Ask for votes in favor, opposed, and abstentions

Executive Director's Report and Q4-2019 Dashboard

Josephine Briggs, MD
Interim Executive Director

- **Priority setting for transition**
- **Review of PCORI's achievements so far**
 - Q4-19 Dashboard Review and End-of-Year Summary
 - A Completed PCORI-Funded Study: From Results to Impact

2019 Dashboard: Quarterly Calendar

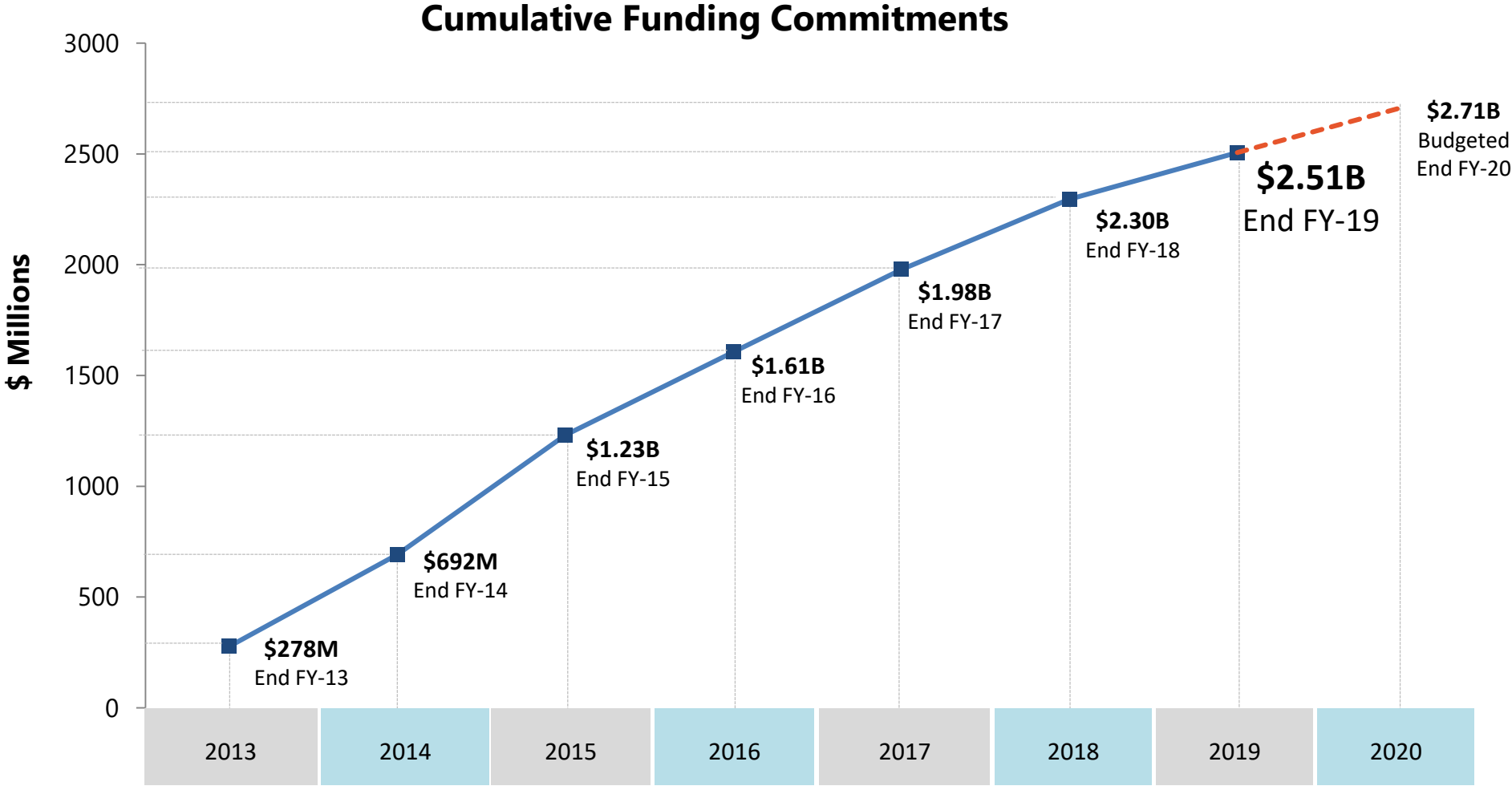


PCORI Board of Governors Dashboard

Fourth Quarter FY-2019 (As of 9/30/2019)



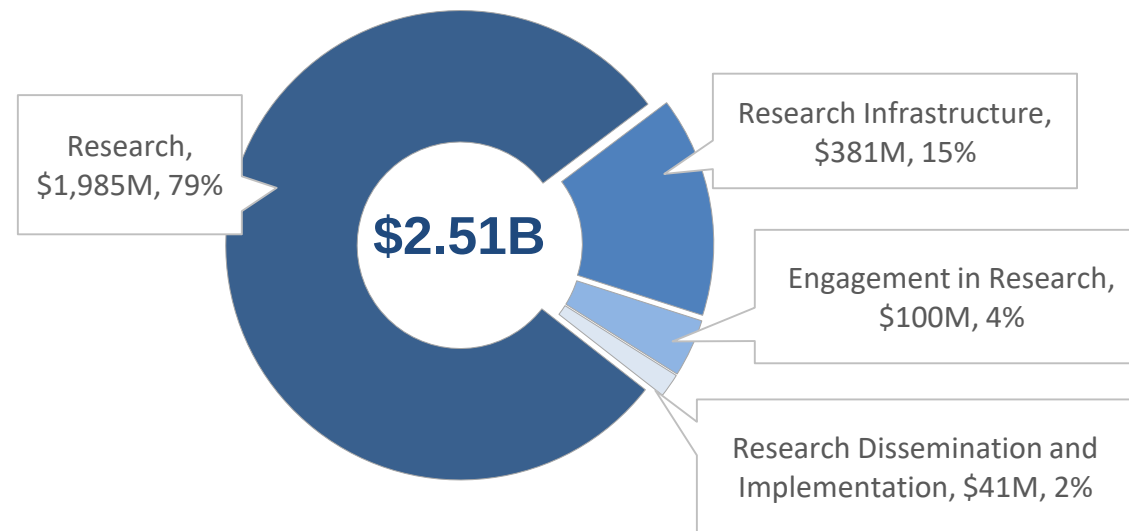
\$2.51B in Cumulative Funding Commitments as of FY-2019



Cumulative Funding Commitments as of FY-2019

All Funding Commitments

as of 9/30/2019



PCORI Board of Governors Dashboard

Fourth Quarter FY-2019 (As of 9/30/2019)

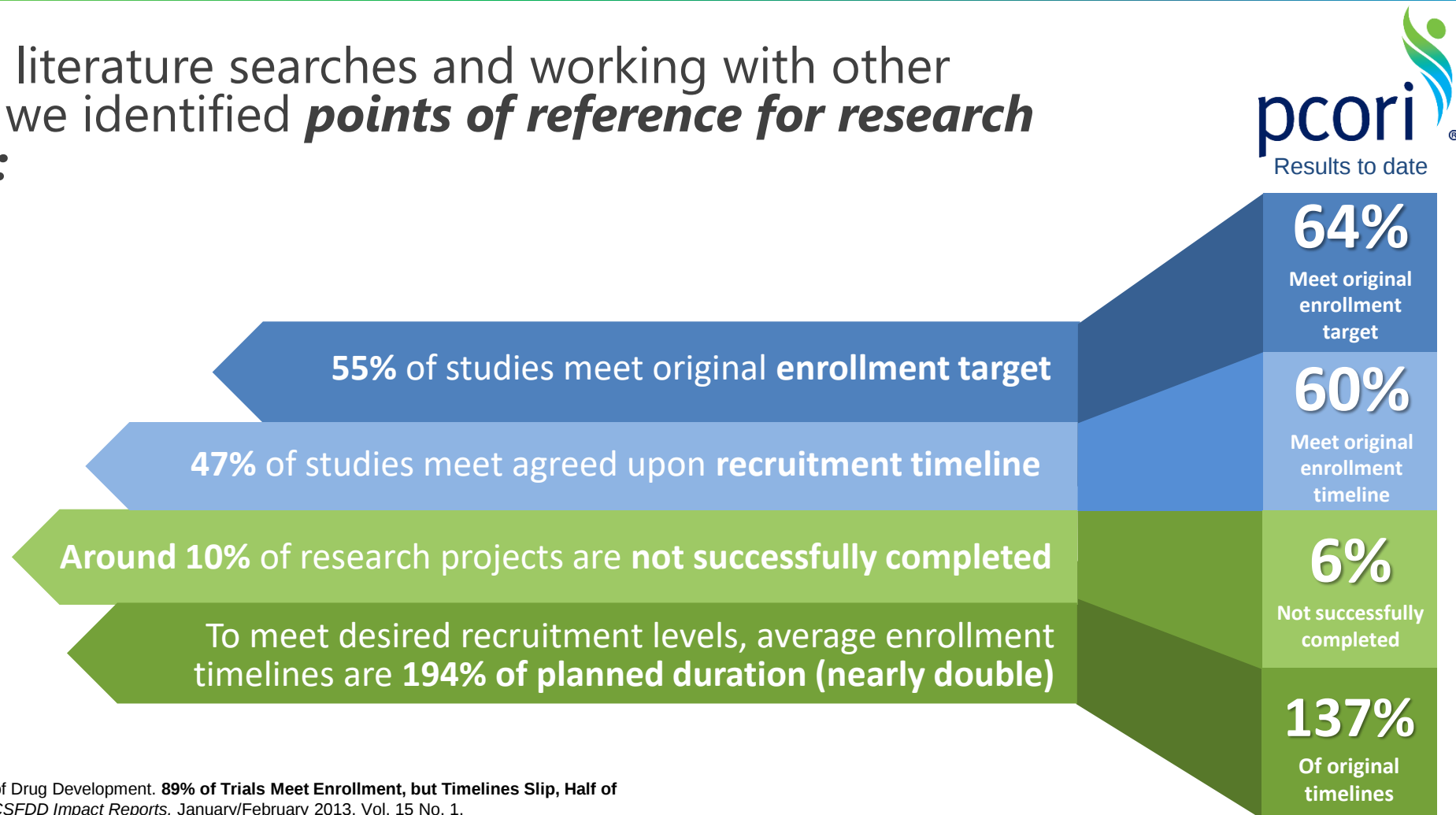


Research Project Performance:

PCORI-funded studies are doing as well or better on recruitment as points of reference



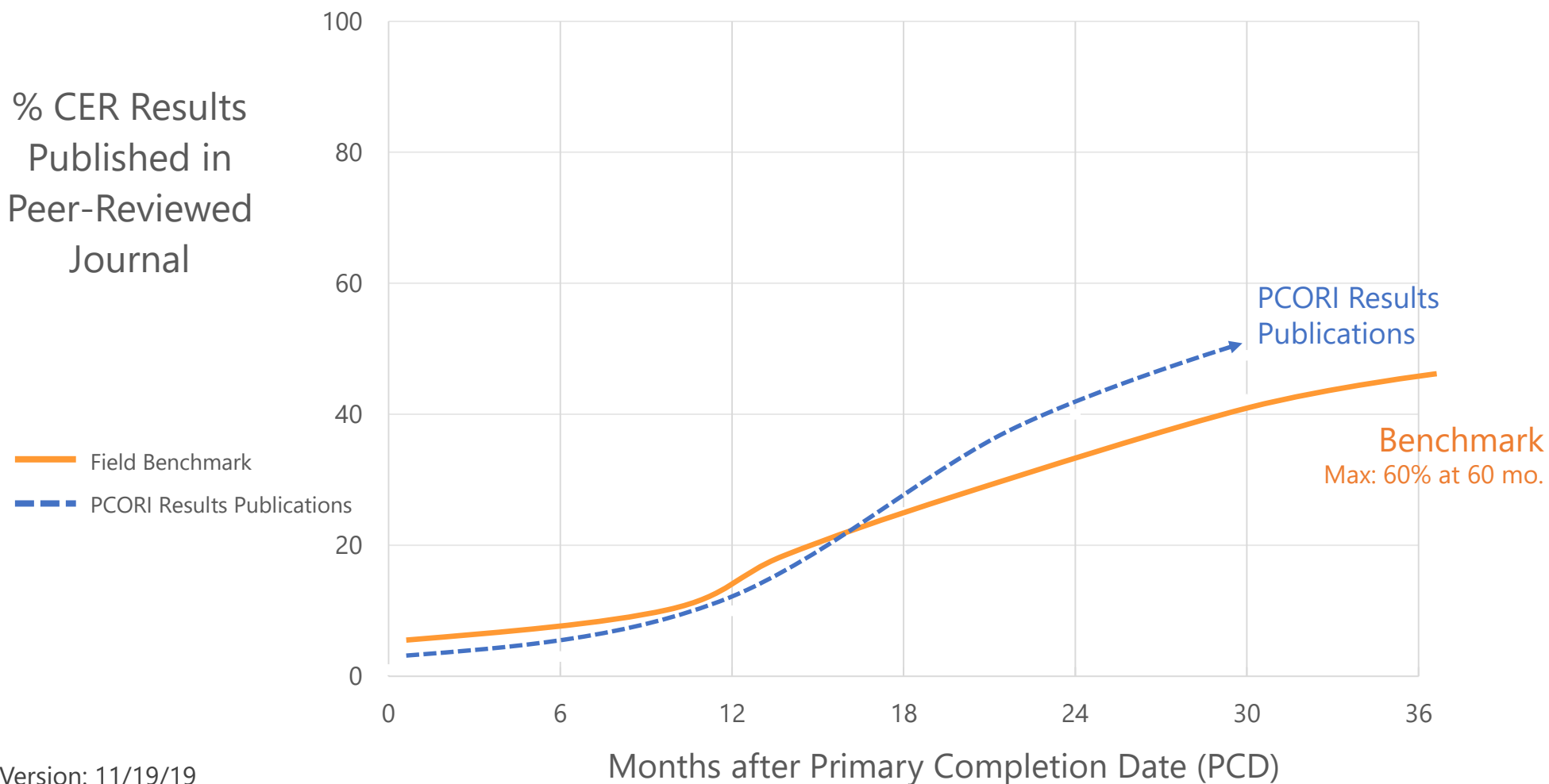
Through literature searches and working with other funders, we identified ***points of reference for research projects:***



Points of Reference:

1. Tufts Center for the Study of Drug Development. **89% of Trials Meet Enrollment, but Timelines Slip, Half of Sites Under-enroll.** *Tufts CSFDD Impact Reports.* January/February 2013, Vol. 15 No. 1.
2. O'Sully, BG, Julious SA, Nicholl J. **A Reinvestigation of Recruitment to Randomised, Controlled, Multicenter Trials: A Review of Trials Funded by Two UK funding Agencies.** *Trials.* Jun 2013.

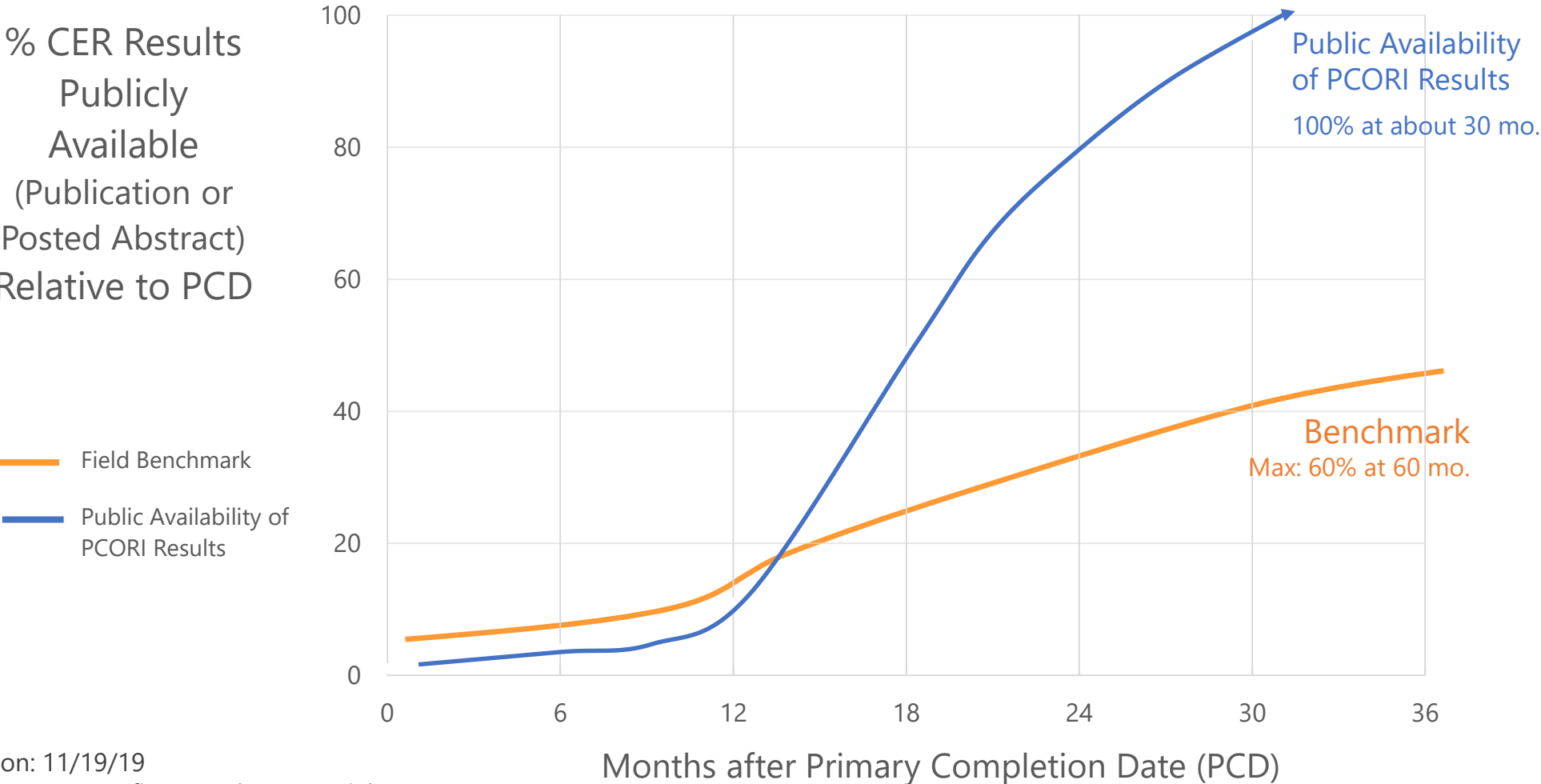
PCORI-Funded Trials Publish Their Results as Quickly as or Faster Than Studies Typically Do



Benchmarks Based on:

- C Riveros, A Dechartres, et al. Timing and Completeness of Trial Results Posted at ClinicalTrials.gov and Published in Journals. Plos One, Dec 2013.
- R Chen, N Desai, H Krumholz, et al. Publication and Reporting of Clinical Trial Results: Cross Sectional Analysis Across Academic Medical Centers. BMJ, Jan 2016.

PCORI's Peer Review Process Results in Complete and Earlier Availability of Results in the Public Domain



Benchmarks Based on:

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PCORI Board of Governors Dashboard

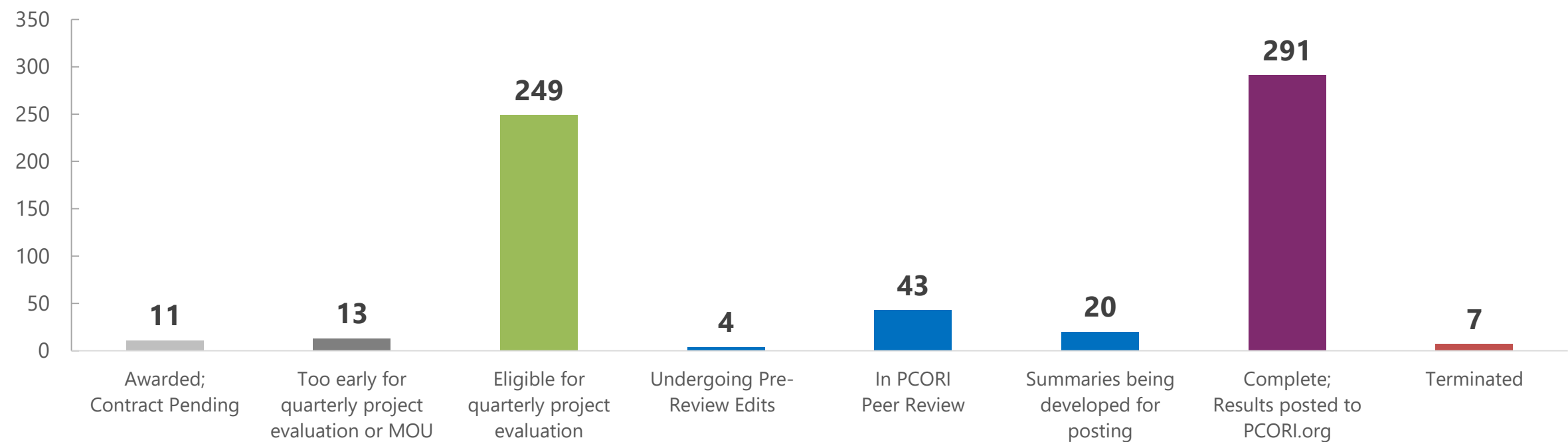
Fourth Quarter FY-2019 (As of 9/30/2019)



Status of Awarded Projects



Current Status of PCORI-Funded Research Projects
N=639, as of Q4-19

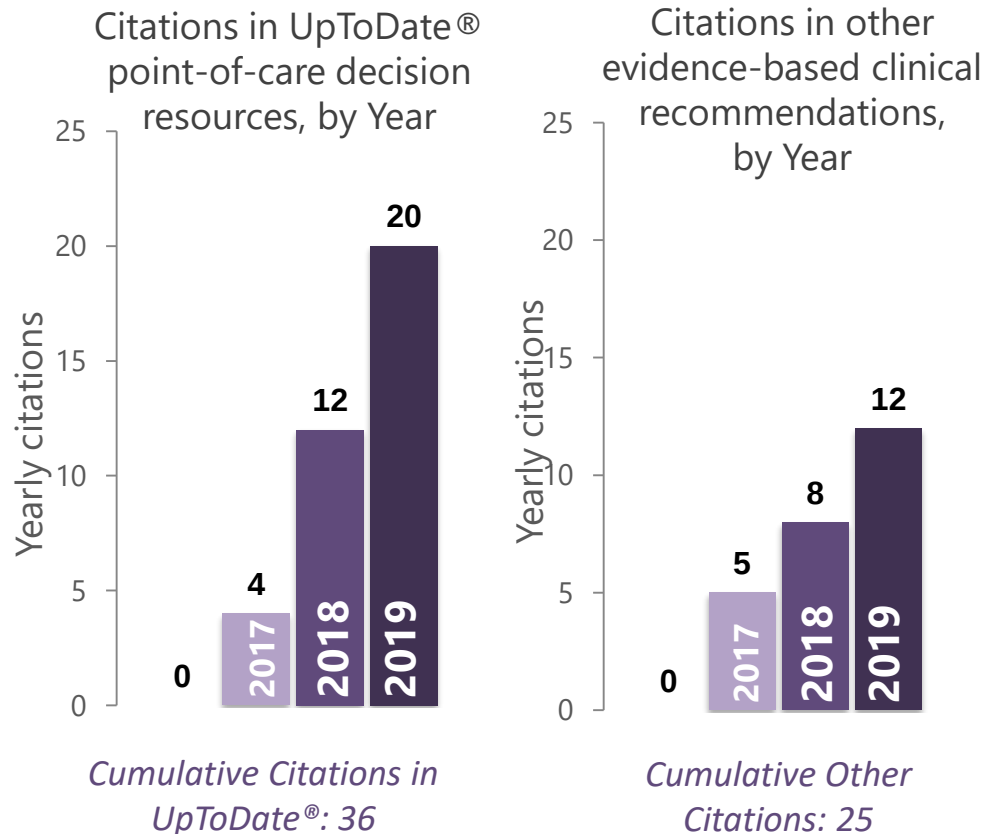


*Includes CER studies, Methods studies, Pilot Awards, Rapid Cycle Research
*Does not include Infrastructure, or D&I awards
*One study was awarded, but contract was not executed
*Does not include studies awarded in Q1-2019

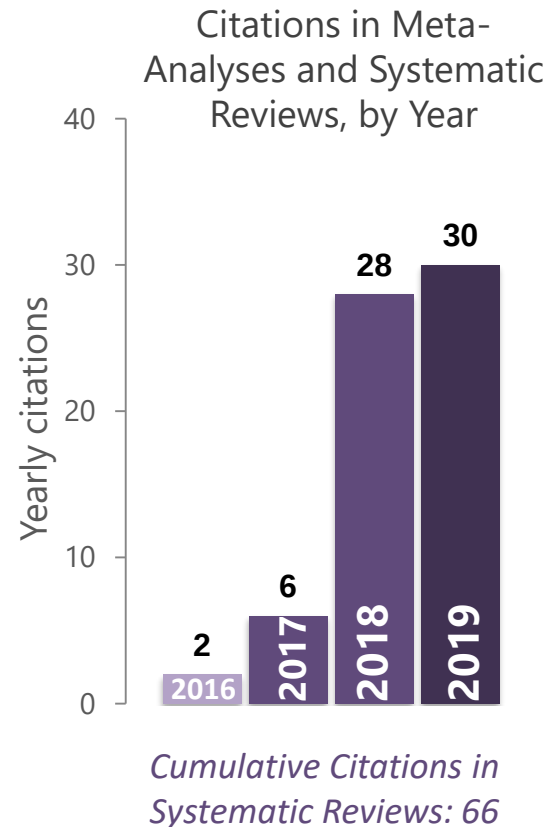
Strong showing on steps toward implementation

(Goal 2: Speed the Uptake and Use of Study Results)

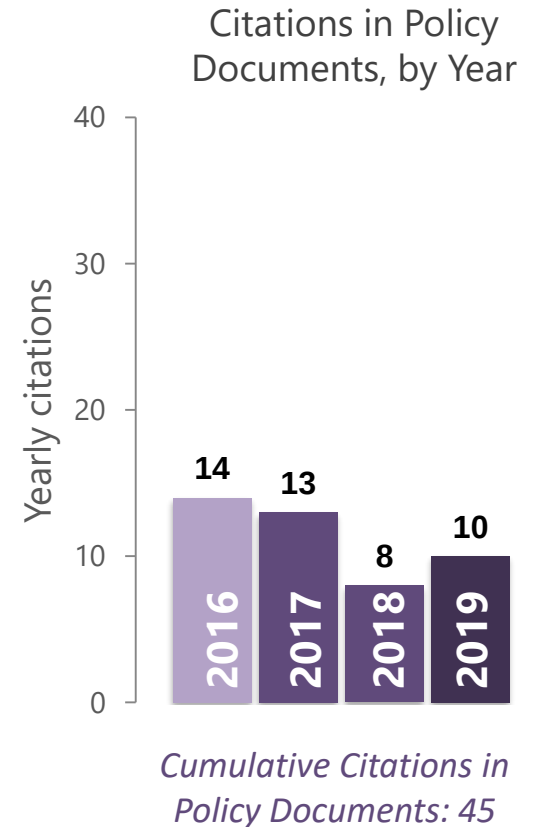
Uptake into Evidence-Based Clinical Recommendations



Uptake into Systematic Reviews Publications

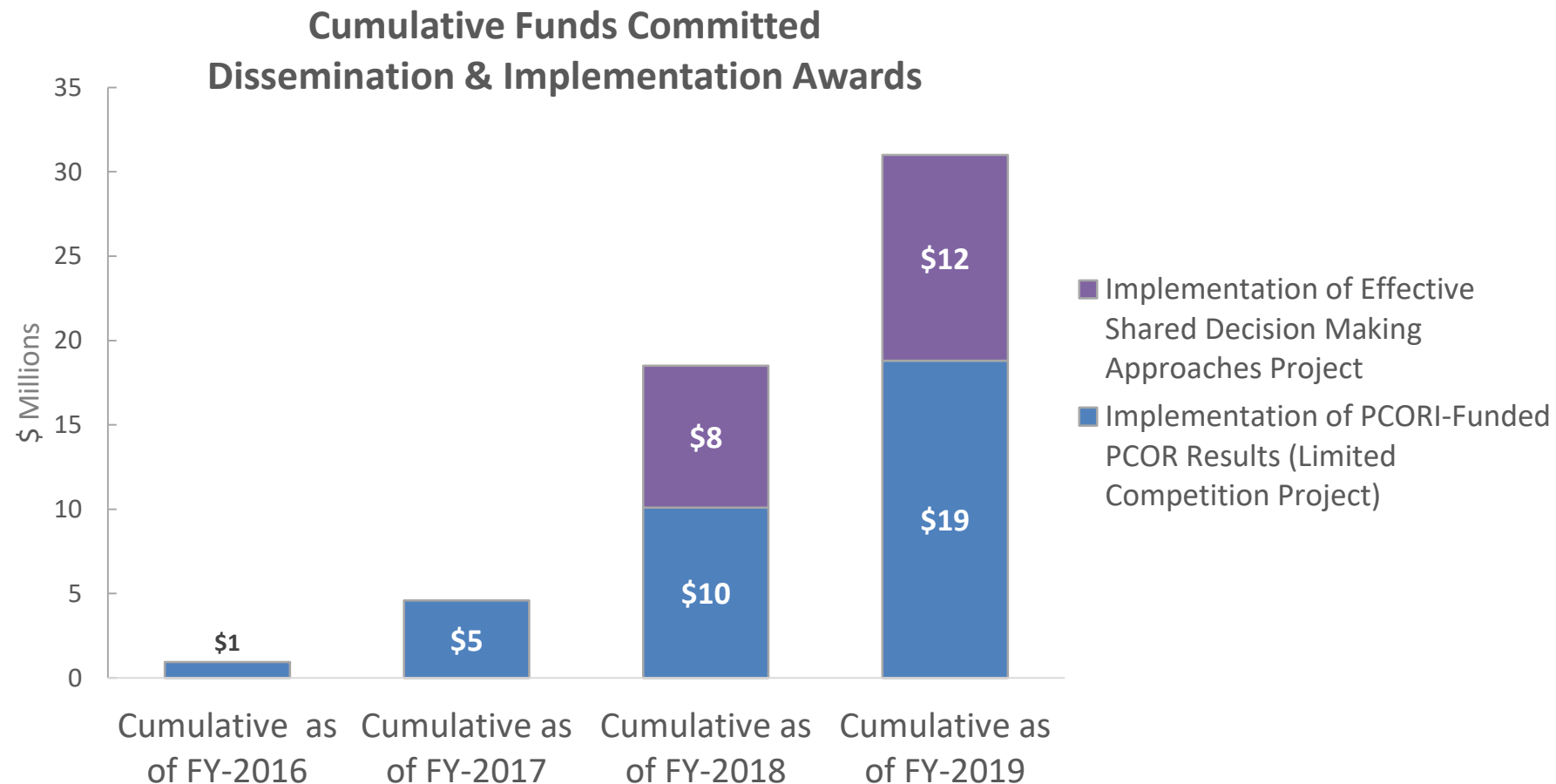


Uptake into Policy Documents



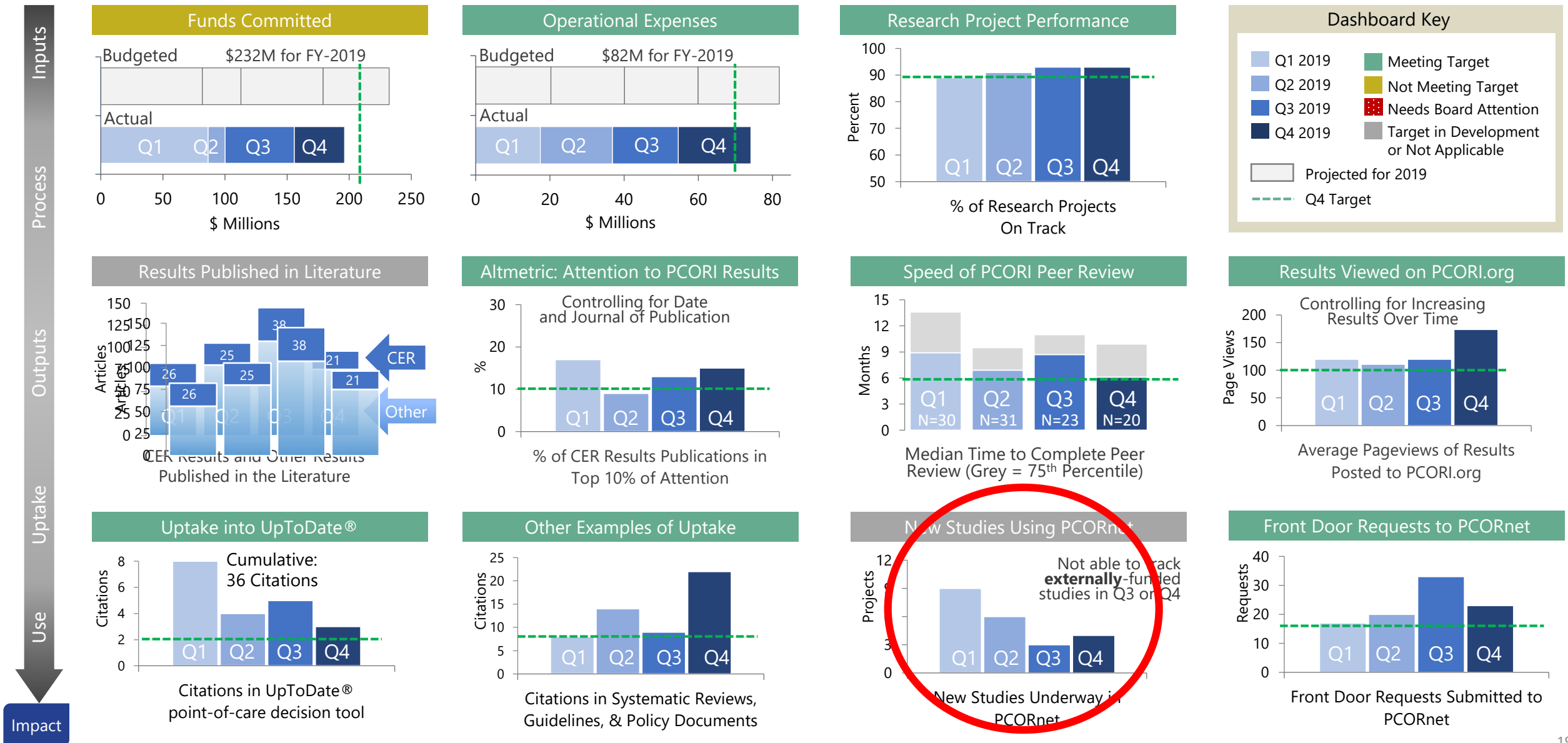
PCORI's program to promote dissemination and implementation is growing as results from portfolio increase

(Goal 2: Speed the Uptake and Use of Research Results)



PCORI Board of Governors Dashboard

Fourth Quarter FY-2019 (As of 9/30/2019)



Increase research using PCORnet: New \$90M NIH-Funded Study Will Utilize PCORnet®

(Goal 3: Influence the Way Research is Done)



- The NIH just announced a \$90 million study that will utilize the PCORnet® infrastructure to conduct a large study more efficiently
- The PREVENTABLE study will test whether statins prevent dementia and other disabilities in older adults without cardiovascular disease
- PREVENTABLE will recruit approximately 20,000 study participants from 40 healthcare systems, and an additional 60 hospitals in the Veterans Affairs Health Network, which is a study partner
- Cost – per patient per year – is about one tenth of typical costs of large randomized trials



Because of the large size of this study, we may be able to identify subgroups of older adults most likely to benefit from taking statins to prevent dementia, disability or cardiovascular disease... the large, diverse study population will also enable us to better evaluate unwanted effects of statins that may put some at risk or reduce their quality of life.

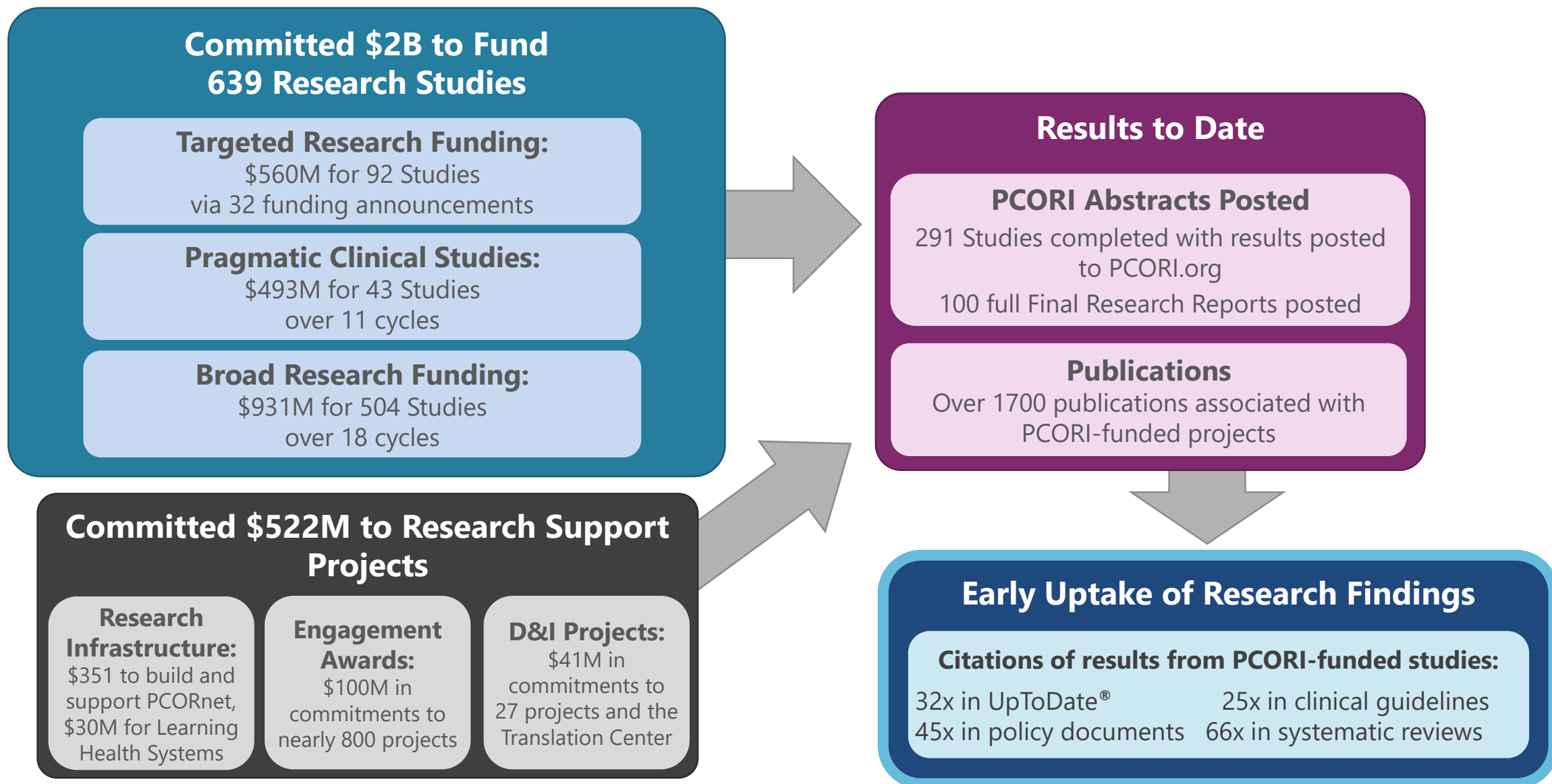
-Susan Ziemann, M.D., Ph.D.

Medical Officer, Division of Geriatrics and Clinical Gerontology, National Institute on Aging (NIA)

Quote from: NIA Press Release. [Could taking statins prevent dementia, disability?](#) Oct 23, 2019.



PCORI at the end of FY-2019: Vision Realized



A Completed PCORI-Funded Study: The Sequence from Results to Impact



Example: Comparing Broad- and Narrow-Spectrum Antibiotics for Children with Ear, Sinus, and Throat Infections

Principal Investigator: Jeffrey Gerber, MD, PhD, The Children's Hospital of Philadelphia

Study Title: Comparative Effectiveness of Broad- versus Narrow-Spectrum Antibiotics for Acute Respiratory Tract Infections in Children

- 1. Results published in JAMA
- 2. Results summaries posted to PCORI.org
- 3. Final Research Report posted to PCORI.org
- 4. Incorporation into PCORI Evidence Updates
- 5. Use by external authorities
- 6. Resulted in a PCORI-funded Implementation Project
- 7. Economic estimates of potential impact and cost-savings

SECTIONS

[Results Summary And Professional Abstract](#)

[Final Research Report](#)

[Related PCORI Dissemination And Implementation Project](#)

[Journal Articles](#)

[More On This Project](#)

[CME/CE Activity](#)

[Evidence For Decisions](#)

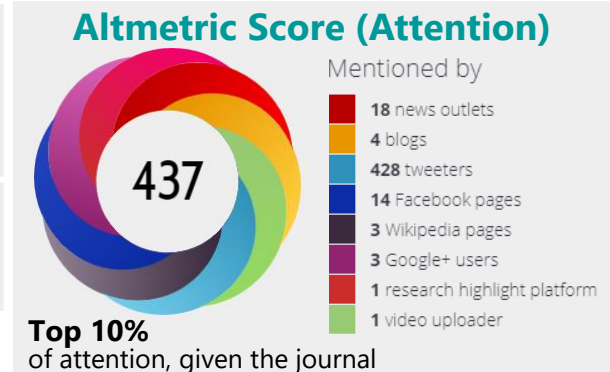
[Peer-Review Summary](#)

[Conflict Of Interest Disclosures](#)

[Project Details](#)

1. For Pediatric Respiratory Infections, Narrow-Spectrum Antibiotics Associated with Better Outcomes than Broad

PCORI Study	Study Title: Comparative Effectiveness of Broad- versus Narrow-Spectrum Antibiotics for Acute Respiratory Tract Infections in Children Principal Investigator: Jeffrey Gerber, MD, PhD, Children’s Hospital of Pennsylvania
Results Publication	Gerber JS, Ross RK, Bryan M, et al. Association of Broad- vs Narrow-Spectrum Antibiotics With Treatment Failure, Adverse Events, and Quality of Life in Children With Acute Respiratory Tract Infections. <i>JAMA</i> . Dec 2017



Summary: Antibiotics are the most common medicines prescribed to children, the majority resulting from respiratory tract infections. Experts recommend starting treatment with narrow-spectrum antibiotics, but guidance is inconsistent, and comparative data has been lacking.

This large retrospective cohort study compared the effectiveness of broad- vs. narrow-spectrum antibiotic treatment for acute respiratory tract infections in children. Investigators worked with patients’ families to identify and gather data on key patient-centered outcomes.

The study found that **narrow-spectrum antibiotics were associated with lower rates of adverse events and higher quality of life** for patients. There were no other differences between clinical or patient-centered outcomes.

“We want to use antimicrobials to treat diseases in the best way possible without creating collateral damage... We know that **parents and pediatricians want more information to help them balance the risks and benefits** of different antibiotics.”

-Dr. Jeffrey Gerber, Principal Investigator
[PCORI Research-in-Action Article](#)

2.PCORI-Funded Study Results ➔Results Summaries



Results Summaries: When studies complete PCORI Peer Review, study results are translated into two clear-language summaries, one for the general public and one for healthcare professionals, and are posted to PCORI.org within 90 days after accepting the final results of the study.

Results Summary for the General Public



PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

RESEARCH SUMMARY

PROJECT INFORMATION

May 2018
(Updated October 2019)

Comparing Broad- and Narrow-Spectrum Antibiotics for Children with Ear, Sinus, and Throat Infections

Principal investigator
Jeffrey Gerber, MD, PhD

Organization
Children's Hospital of Philadelphia

What was the research about?
Antibiotics are the most commonly prescribed medicine in children. Narrow-spectrum antibiotics target a few types of bacteria. Broad-spectrum antibiotics target many types of bacteria. Both types work well to treat infections. But using broad-spectrum antibiotics when they're not needed can create antibiotic-resistant bacteria that are hard to treat. They may also have side effects, such as diarrhea or rash.

In the first, the research team looked at health records for 30,159 children who used broad- or narrow-spectrum antibiotics to treat ear, sinus, or throat infections. Of the children, 63 percent were white,

Professional Abstract Results Summary

Professional Abstract

Objective
To compare the relative effectiveness of narrow- and broad-spectrum antibiotics in treating acute respiratory tract infections (ARTIs) in children

Study Design

Design Elements	Description
Design	Observational: cohort study
Population	Retrospective cohort: 30,159 children ages 6 months to 12 years with ARTIs Prospective cohort: 2,472 children ages 6 months to 12 years with ARTIs
Interventions/	- Narrow-spectrum antibiotics - Broad-spectrum antibiotics
Outcomes	Primary outcome: treatment failure (primary), occurrence

This is currently the most-viewed results page on the PCORI website, with 3,700 pageviews

3. PCORI-Funded Study Results → Final Research Report

Final Research Report (FRR): The comprehensive, final peer-reviewed report of the funded study, usually equivalent to the length and content of 3-5 journal articles, which is posted to the project page

The FRR for the results of this study is a 93-page, open-access write-up of the study, including detailed methods, results, tables, and figures



Final Research Report
View this project's [final research report](#).

Gerber J, Ross R, Bryan M., et al. 2018. Comparing Broad- and Narrow-Spectrum Antibiotics for Children with Ear, Sinus, and Throat Infections. Washington, DC: Patient-Centered Outcomes Research Institute (PCORI). <https://doi.org/10.25302/8.2018.CE.13047279>

This is currently the most downloaded FRR on the PCORI website, with 143 downloads

4. PCORI-Funded Study Results → Evidence Updates

Evidence Updates: PCORI presents findings from systematic reviews and some of our funded research studies in concise, accessible formats. Evidence Updates are created and disseminated in collaboration with patient, provider, or other organizations.

Evidence Update for Parents



PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE
EVIDENCE UPDATE

OCTOBER 2018

Choosing an Antibiotic for Your Child's Ear, Nose, or Throat Infection



New research findings can help you and your child's clinician figure out the best option for treating your child's infection.

When bacteria cause your child's ear, nose, or throat infection, your clinician will suggest one of two kinds of antibiotics. One kind is **narrow-spectrum antibiotics**. These medicines fight just the bacteria that are most likely causing your child's ear, nose, or throat infection. Examples are amoxicillin or Amoxil®. The other kind is **broad-spectrum antibiotics**. These medicines fight many kinds of bacteria at the same time. Examples are azithromycin or Z-Pak®.



FINDINGS FROM A RECENT STUDY FUNDED BY PCORI

In this large study, narrow-spectrum antibiotics worked just as well as broad-spectrum antibiotics at killing the bacteria within three days. But children taking narrow-spectrum antibiotics had fewer side effects like diarrhea and vomiting than children taking broad-spectrum antibiotics.

Evidence Update for Clinicians



PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE
EVIDENCE UPDATE

OCTOBER 2018



Comparing Narrow- vs. Broad-Spectrum Antibiotics for Common Infections in Children

The choice of antibiotic to treat acute bacterial upper respiratory tract infections in children can affect both symptom resolution and the risk of side effects such as diarrhea and vomiting.

The findings of a recent PCORI-funded study published in *JAMA*¹ can help clinicians treating children for acute respiratory tract infections (ARTIs)—including acute otitis media, Group A streptococcal pharyngitis, and acute sinusitis—make decisions with parents about the medicine that is best for the child. The study, led by Jeffrey Gerber, a pediatrician and researcher at the Children's Hospital of Philadelphia, included 30,086 children ages 6 months to 12 years taking narrow- and broad-spectrum antibiotics to treat ARTIs.



THE FINDINGS

A PCORI-funded study published in *JAMA*¹ found that narrow-spectrum antibiotics such as amoxicillin are just as effective as broad-spectrum alternatives for treating acute respiratory tract infections (ARTIs) in children ages 6 months to 12 years but have a lower risk of side effects.

5. PCORI Funded Study Results



Uptake and Use by External Groups

Uptake into External Resources: The Urgent Care Association's Antibiotic Stewardship Council has incorporated results from this study into their antibiotic stewardship resources. They link directly to the Clinician-Focused Summary and the Parent-Focused Summary

- Urgent Care Association. **Antibiotic Stewardship Resources.** <https://www.ucaoa.org/Resources/Antibiotic-Stewardship>; Accessed 11/22/2019

Uptake into Wikipedia: Results from this study are cited in 3 Wikipedia pages:



- [Antimicrobial resistance](#)
- [Broad-spectrum antibiotic](#)
- [Upper Respiratory tract infection](#)

From [Wikipedia](#), the free encyclopedia
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[Attribution-Share Alike 3.0 Unreported](#)

6. PCORI-Funded Study Results → Implementation Project

Implementation Projects: PCORI funds implementation projects that incorporate active, multicomponent strategies that will lead to uptake and integration of PCORI-funded evidence into real-world practice settings

- **Title:** Broad Implementation of Outpatient Stewardship (BIOS) Study
- **Principal Investigator:** Jeffrey Gerber, MD, PhD, The Children's Hospital of Philadelphia
- This implementation project will **use tested strategies to improve antibiotic prescribing** for more than 350,000 children across 5 health systems in 3 states, including all frontline providers in a wide mix of outpatient settings
- Intervention includes clinician education on guidelines, audit and feedback reports on prescribing, practice facilitation, and practice champions
- The project will evaluate impacts on provider practice (changes in appropriate antibiotic prescribing rates) and intervention delivery

Broad Implementation of Outpatient Stewardship (BIOS) Study

[Sign Up for Updates to This Study](#)



Project Summary

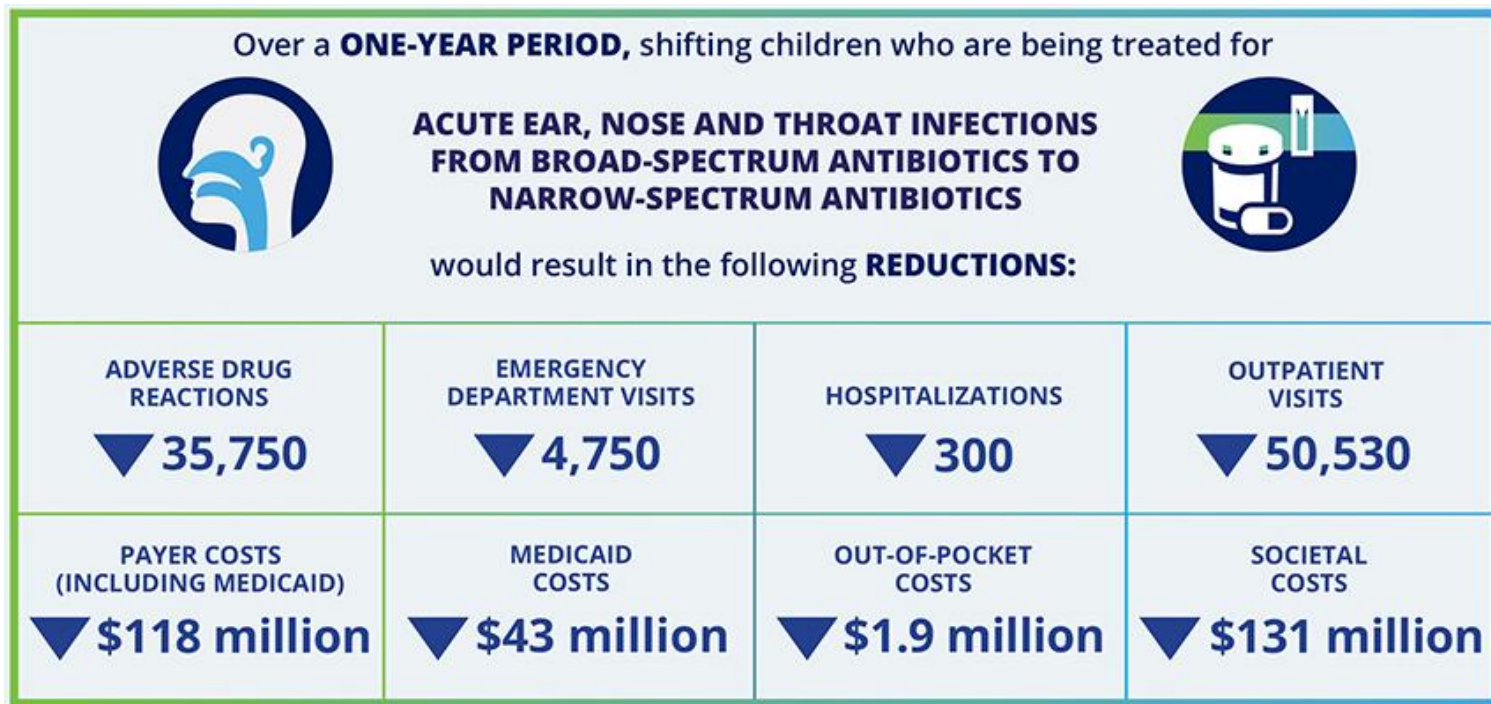
This project focuses on implementing findings from the [PCORI-funded study](#) on use of narrow-spectrum versus broad-spectrum antibiotics to treat children's acute respiratory tract infections (Principal Investigator: Jeffrey Gerber), in the context of existing evidence on this topic.

1. What is the eligible evidence proposed for implementation?

Antibiotics are the most common medications prescribed for children. Although antibiotics can be lifesaving drugs, up to half of all antibiotic use is inappropriate. Excessive antibiotic use can lead to antibiotic-resistant bacteria, which sicken at least 2 million Americans each year and are hard to treat. Prior PCORI-funded work by this project team compared the effectiveness of broad-spectrum (like cephalosporins) vs. narrow-spectrum (like amoxicillin) antibiotics in children treated for three common

7. PCORI-Funded Study Results → Impact

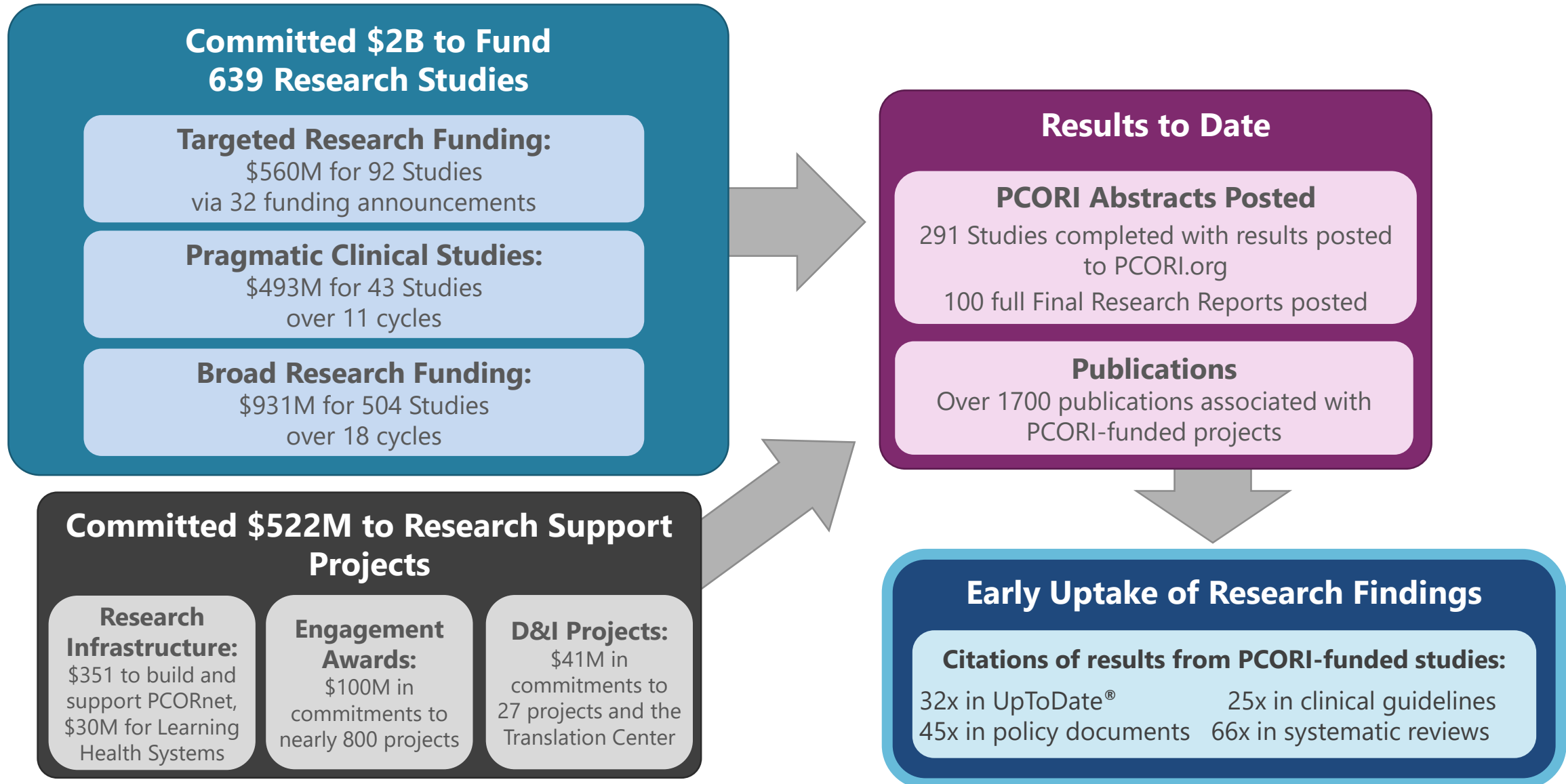
Evidence with Impact: PCORI provides examples of how PCORI-funded studies can help patients choose care that is right for them, improving outcomes they care about, reducing unnecessary treatments and costs.



Gauging the Potential Impact of Results

- If findings from this study were widely implemented, there would be an additional 2.7 million children who would receive narrow rather than broad-spectrum antibiotics, resulting in **\$131M reduction in societal costs per year, including healthcare savings of about \$118M (payer costs, including Medicaid)**
- Visit [PCORI.org/Impact](https://www.pcori.org/Impact) for more impact analyses

PCORI at the end of FY-2019: Vision Realized



Discussion Questions

- What are important short-term goals for an interim director?
- How can PCORI staff help the Board prepare for planning PCORI 2.0?

Engagement: What We've Learned

Kristin L. Carman, PhD, MA
Director, Public and Patient Engagement

Laura Forsythe, PhD, MPH
Director, Evaluation and Analysis

Lia Hotchkiss, MPH
Director, Engagement Awards Program

Outline



- Background: PCORI's mandate and engagement as a foundation
- What we've learned: The difference engagement has made
- What's next: Implications for the future

Background

The Patient-Centered Outcomes Research Institute: Established to Meet Stakeholders' Needs

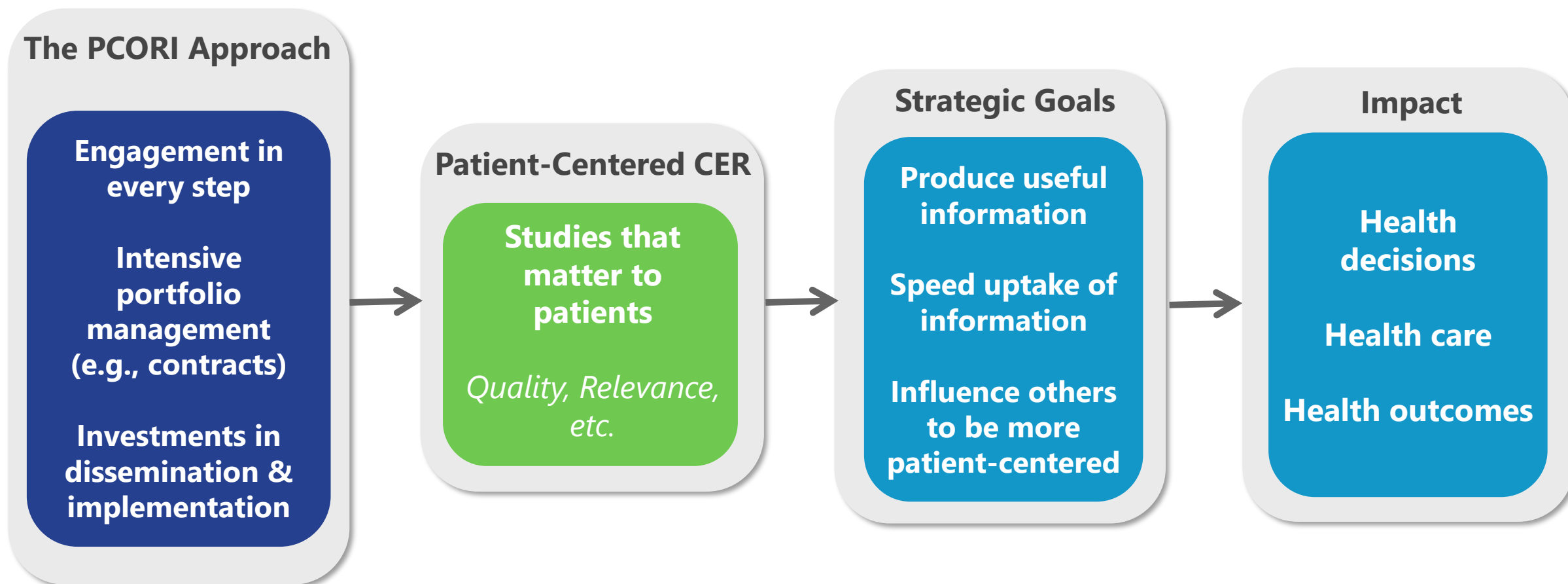


“The purpose of the Institute is to **assist patients, clinicians, purchasers, and policy-makers in making informed health decisions by advancing the quality and relevance of evidence** concerning the manner in which diseases, disorders, and other health conditions can effectively and appropriately be prevented, diagnosed, treated, monitored, and managed through research and evidence synthesis... and the **dissemination** of research findings with respect to the relative health outcomes, clinical effectiveness, and appropriateness of the medical treatments, services...”

—from PCORI’s authorizing legislation



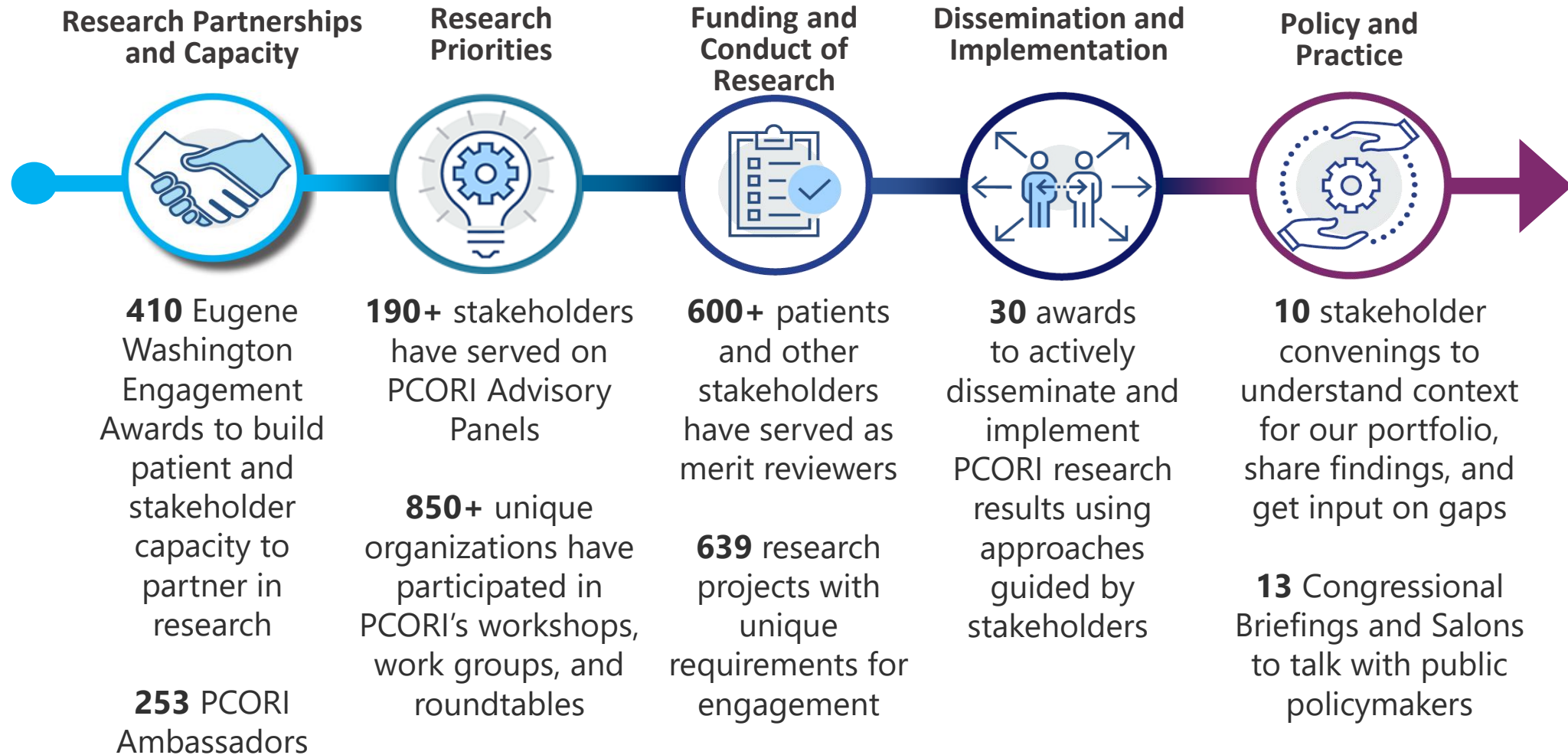
The Path to Impact: PCORI's Conceptual Model



Engagement: A Foundation for Everything PCORI Does



PCORI Creates Opportunities for Engagement at Every Step of the Research Process



Findings synthesized across efforts to build evidence and inform practice



What We've Learned

Engagement Makes Meaningful Differences in Research Projects



Core themes emerged across multiple studies of engagement

Engagement influenced...

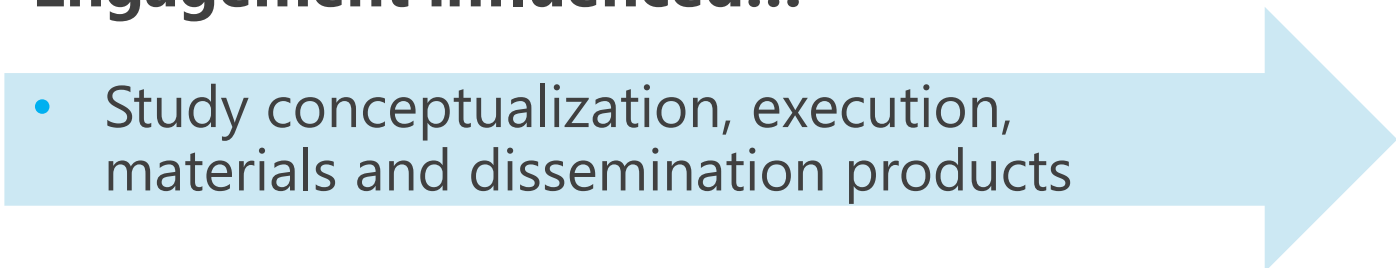
- Study conceptualization, execution, materials and dissemination products
- Carrying out study tasks
- Engagement design and practice
- Researchers' understanding of patients, clinicians, and health care organizations

For example, in one recent study about engagement in PCORI-funded research projects, nearly 400 distinct examples of engagement influence were analyzed from in-depth interviews with 60 projects

Engagement Makes Meaningful Differences in Research Projects

Engagement influenced...

- Study conceptualization, execution, materials and dissemination products
- Carrying out study tasks
- Engagement design and practice
- Researchers' understanding of patients, clinicians, and health care organizations



Engagement influences **all aspects** of CER projects

Research focus
Research design
Intervention tailoring/delivery
Recruitment/retention
Data collection/measures
Data analysis
Dissemination

Engagement Makes Meaningful Differences in Research Projects

Engagement influenced...

- Study conceptualization, execution, materials and dissemination products
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Engagement Influences Research Focus

- Identification of topic or project
- Formulation or expansion of research aims or questions
- Choice of comparator(s)
- Determination of research outcomes (primary and secondary)

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Comparing the Effectiveness of Nonsurgical Treatments for Lumbar Spinal Stenosis (LSS) in Reducing Pain and Increasing Walking Ability

Included measures of physical activity and walking capacity as primary outcome measures because stakeholders and patients stressed the importance of maintaining their independence.

Engagement Influences Research Focus

- Identification of topic or project
- Formulation or expansion of research aims or questions
- Choice of comparator(s)
- Determination of research outcomes (primary and secondary)

Comparing Different Treatments for People Who Have Had a Stroke – The PROSPER Study

Modified primary outcomes based on patient priorities to include home time out of hospital.

“Yes, stroke survivors were concerned about long-term survival, but they were equally concerned about the quality of that survival and the potential for recovery beyond 3 months or 1 year (i.e., the typical time points in stroke outcomes research)... So we revisited our aims, overhauled our data collection plan, and ensured that our goals were not only informed by patients but also aligned with the issues that patients cared about the most.”

- O'Brien E., 2014, *Circ Cardiovasc Qual Outcomes*

Engagement Influences Research Design

- Practical guidance on how to carry out the research
- Choice of design (e.g., delayed start, mixed methods)
- Study participant allocation and randomization designs
- Broader inclusion and less restrictive exclusion criteria

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Comparing Clinic and Home-Based Exercise Programs to Help Adults with Multiple Sclerosis (The TEAMS Study)

Chose to randomize by clinics (cluster randomization) rather than individuals to prevent treatment crossover, based on stakeholder feedback about patients participating together in clinic-based support groups.

Engagement Influences Recruitment and Retention

- Designing outreach and recruitment strategies (e.g., developing consent materials, setting compensation)
- Anticipating barriers (e.g., addressing factors to improve participation among hard-to-reach populations)
- Solving unanticipated problems
- Participating in recruitment, enrollment, and retention activities

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Comparing Broad- and Narrow-Spectrum Antibiotics for Children with Ear, Sinus, and Throat Infections

Partners helped to create the consent script used to recruit parent participants.

Engagement Influences Recruitment and Retention



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Tailoring Resources to Help Children and Parents Manage Type 1 Diabetes

Stakeholders provided input that helped the study appeal to families who traditionally experience disparities in diabetes control & spoke in favor of in-person recruitment.

"As a result, minority families were enrolled in numbers proportionate to our sites' population demographics and as good as or better than similar recently published trials."

- Fiallo-Scharer R., 2017, Contemp Clin Trials

Linking the Influence of Engagement to Effects on the Research Projects

Influence on Study Conduct

- Discrete decisions, events, behaviors, strategies

Effects on the Projects

- How specific influences shape the project (more intermediate)
- Generally focuses on practical aspects

Ways that Engagement Shapes PCORI-Funded Research Projects



User-Orientation & Acceptability:

studies in which patients and clinicians will be willing to participate based on burden, usability, and alignment with preferences, values, and needs

Ways that Engagement Shapes PCORI-Funded Research Projects



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Training Doctors to Discuss Goals of Care with Patients Who Have Advanced Cancer

Patient partners ensured that survey response burden for outcome measures was minimized for people with advanced cancer, who fatigue easily

Oncologists informed understanding of the practice landscape and refined and the intervention (e.g., addressed concerns about time involved, demonstrating respect for oncologist-patient relationship)

"Rates of physician participation and retention were higher than usual in our intervention, suggesting that involving physician stakeholders, though slower in the design phase, created a more relevant study and increased buy-in."

- Solomon, 2017, Patient

Ways that Engagement Shapes PCORI-Funded Research Projects



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studies in which patients and clinicians will be willing to participate based on burden, usability, and alignment with preferences, values, and needs

Feasibility:

interventions, enrollment, and data collection that are more doable in real-world settings

Ways that Engagement Shapes PCORI-Funded Research Projects



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*"To our knowledge, this is the largest telerehabilitation trial ever conducted on people with MS anywhere in the world. In fact, there are very few studies of this magnitude in any area of MS research. If we didn't have this front end, patient engagement structure, which PCORI requires in the application process, we'd probably be behind on recruitment. **This study was set up in such a way that there were built-in mechanisms for ensuring that you reach the finish line** with the appropriate sample size that will allow us to take the study to the next level."*

James Rimmer, PhD, MA, PI of The TEAMS study
University of Alabama at Birmingham

Ways that Engagement Shapes PCORI- Funded Research Projects



User-Orientation & Acceptability:

studies in which patients and clinicians will be willing to participate based on burden, usability, and alignment with preferences, values, and needs

Feasibility:

interventions, enrollment, and data collection that are more doable in real-world settings

Quality:

study rigor, comprehensiveness, and quality of materials and products

Relevance:

results applicable and important for decision-making

Engagement Scope & Quality:

engagement processes are effective, and stakeholders are well equipped

Patients & Stakeholders

- More knowledge and enthusiasm for research
- Developed skills and professional opportunities
- Improved personal health and healthcare
- New or better relationships
- Feeling of making a difference

Communities

- Built trust
- Increased research capacity
- Strengthened relationships among stakeholders
- Increased awareness of different stakeholder perspectives

Researchers

- Deeper understanding of real-world experiences and concerns of their study populations
- More knowledge about engagement
- Commitment to engagement in the future



PCORI's Approach to Engagement is Helping Achieve Our Third Strategic Goal of Influencing the Culture of Research



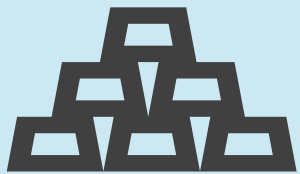
PCORI is credited with inspiring change at other organizations and institutions

Specific examples are described in more detail at <https://www.pcori.org/engagement/influencing-culture-research>

- Seed funding for partnerships
- Training of students, fellows, and partners
- Patient reviewers
- Requirement to involve patients & stakeholders in strategic research planning
- Standing patient & stakeholder advisory boards
- Policies to support engagement in research
- Journal policies for person-centered language
- Adoption of patient-centered approaches by federal agencies, academic institutions, and funders

Engagement Can Be Challenging

Infrastructure and Resources



- Knowledge
- Skills & Experience
- Money
- Time

People and Teams



- Access
- Ensuring Diversity & Inclusivity
- Relationships & Communication

Organizations



- Competing Priorities
- Contracting Policies
- IRBs

Balancing Views and Priorities



- Differing Perspectives
- Value Conflicts
- Internal vs. External Validity

In Sum: What Has PCORI Accomplished in Engagement?

Opportunities
for Engagement

Guidance

Portfolio of
Projects and
Research

Science of
Engagement

Culture Change

In all aspects of
the research
process

Meetings & Events

Type of Event
Stakeholder Workshops ▼ **Go**

AUG 22

PCORI Stakeholder Webinar on Cervical Ripening in the Outpatient Setting: A Systematic Review
PCORI hosted a stakeholder webinar on cervical ripening in the outpatient setting, which is the topic for a new systematic evidence review commissioned via the... [Read More](#)

AUG 20

PCORI Stakeholder Webinar on Radiation Therapy for Brain Metastases: A Systematic Review
PCORI hosted a stakeholder webinar on radiation therapy for brain metastases, which is the topic for a new systematic evidence review commissioned via the Agency... [Read More](#)

JUL 09

PCORI Stakeholder Webinar on Interventions for Dyspnea in Patients with Advanced Cancer: A Systematic Review
PCORI hosted a stakeholder webinar on interventions for dyspnea in patients with advanced cancer, which is the topic for a new systematic evidence review commissioned...

**PATIENT-CENTERED OUTCOMES
RESEARCH INSTITUTE**
1838 L STREET NW, SUITE 900
WASHINGTON, DC 20036
202.627.7700

Seeking Reviewers FOR SUMMARIES OF RESEARCH



Are you the parent of a child newly diagnosed with Duarte galactosemia (DG)?
If yes, Patient-Centered Outcomes Research Institute, or PCORI, would like to invite you to review a short summary from a recently completed study about DG. We believe you provide a valuable perspective that can improve these materials!

Who is PCORI?
PCORI is a national nonprofit, nongovernmental organization. PCORI creates easy-to-read summaries of research findings for the general public. PCORI asks patients and caregivers to review summaries to make sure they are written in a way that is easy to understand and useful.

What Would I Need to Do?
If you agree to help, PCORI will send you a 1.5-page summary to read and a form with questions asking how clear or confusing you found the summary. After you have completed your review, you would send your responses back to PCORI.

Time requirement It usually takes about 30-40 minutes to review the summary and complete the form.	Timeline The abstract will be available to review mid July. You will have 2 business days to complete your review.	Compensation You will receive \$75 for reviewing the summary and completing and returning the form.
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In Sum: What Has PCORI Accomplished in Engagement?

Opportunities
for Engagement

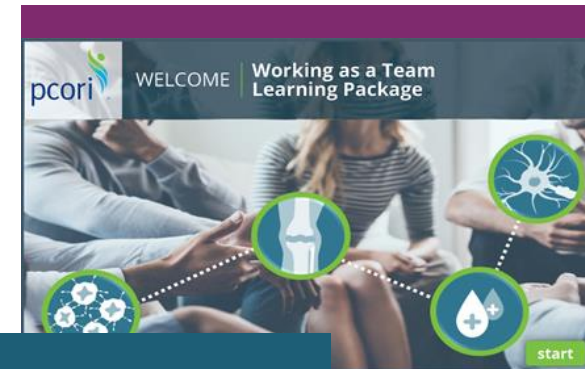
Guidance

Portfolio of
Projects and
Research

Science of
Engagement

Culture Change

**Initially general,
non-directive
supports;
increasingly more
structured and
tailored and based
on developing
evidence**



In Sum: What Has PCORI Accomplished in Engagement?

Opportunities
for Engagement

Guidance

**Portfolio of
Projects and
Research**

Science of
Engagement

Culture Change

In Sum: What Has PCORI Accomplished in Engagement?

Opportunities
for Engagement

Guidance

Portfolio of
Projects and
Research

**Science of
Engagement**

Culture Change

**Body of evidence
from large-scale
laboratory of
innovation**

In Sum: What Has PCORI Accomplished in Engagement?

Opportunities
for Engagement

Guidance

Portfolio of
Projects and
Research

Science of
Engagement

Culture Change

What's Next

Future Actions to Build on PCORI's Accomplishments

Opportunities for Engagement

Expanded approaches and mechanisms to ensure participants are diverse and representative

Guidance

Update Rubric; explore opportunities to expand standards and criteria for engagement

Portfolio of Projects and Research

Make engagement easier and more efficient through portfolio management and support

Science of Engagement

Implement innovative methods, including exploring new funding opportunities

Develop and use validated measures of engagement

Culture Change

Spread learnings about engagement

Discussion of the Implications for Research and Practice



- What do our findings suggest about PCORI 2.0 in terms of engagement science, policy, and practice?
- What are the priorities for revamping or developing capacity, infrastructure, resources, and tools for engagement science and practice?
- What additional types or sources of evidence are needed?

Selected References for Further Reading



- Hemphill R, Forsythe LP, Heckert AL, Amolegbe A, Maurer M, Carman K, Mangrum R, Stewart L, Fearon N, and Esmail L. **What Motivates Patients and Caregivers to Engage in Health Research and How Engagement Affects Their Lives: Qualitative Survey Findings.** Health Expectations. 2019; 00: 1– 9. <https://doi.org/10.1111/hex.12979>
- Mauer, Maureen. **"Understanding the Influence and Impact of Engagement in PCORI-Funded Studies: A Qualitative Study."** PCORI Annual Meeting 2019, 19 September 2019, Washington D.C.. Plenary Presentation: How Engagement Is Making Research More Useful. Slides: <https://www.pcori.org/sites/default/files/PAM19-Lunch-Plenary-Engagement-Making-Research-Useful-Maurer-Presentation-Slides.pdf>.
- Forsythe LP, Carman KL, Szydlowski V, Fayish L, Davidson L, Hickam DH, Hall C, Bhat G, Neu D, Stewart L, Jalowsky M, Aronson N, Anyanwu CU. **Patient Engagement In Research: Early Findings From The Patient-Centered Outcomes Research Institute.** Health Aff (Millwood). 2019 Mar;38(3):359-367.
- Boivin A, Richards T, Forsythe L, Grégoire A, L'Espérance A, Abelson J, Carman KL. **Evaluating patient and public involvement in research.** BMJ. 2018 Dec 6;363:k5147.
- Forsythe LP, Frank LB, Tafari AT, Cohen SS, Lauer M, Clauser S, Goertz C, Schrandt S. **Unique Review Criteria and Patient and Stakeholder Reviewers: Analysis of PCORI's Approach to Research Funding.** Value Health. 2018 Oct;21(10):1152-1160.
- Sheridan S, Schrandt S, Forsythe L, Hilliard TS, Paez KA; Advisory Panel on Patient Engagement (2013 inaugural panel). **The PCORI Engagement Rubric: Promising Practices for Partnering in Research.** Ann Fam Med. 2017 Mar;15(2):165-170.

BREAK

We will return in 15 minutes



Join the conversation on Twitter via
@PCORI

PCORI's Cancer Portfolio

Neeraj Arora, PhD

Associate Director

Healthcare Delivery & Disparities Research

Sarah Daugherty, PhD, MPH

Senior Program Officer

Clinical Effectiveness & Decision Science

Presentation Outline



- Background
- Overview of PCORI's Cancer CER Portfolio
- Study Findings to Date
- Summary and Next Steps

Background

- 1.6 million people in the U.S. are diagnosed with cancer every year
- By 2026, there will be over 20 million individuals living with a cancer history
 - More than 2/3 would be 65 years or older
- Cancer treatment trials typically exclude elderly patients or those with comorbid health conditions
- Family caregivers often face a significant burden caring for cancer patients
- Care that is patient-centered, evidence-based, and well-coordinated has been identified as a goal for high quality cancer care (IOM, 2013)

PCORI's Investment in Cancer Research



PCORI HAS AWARDED

\$313 MILLION **80** CER studies
TO FUND

on cancer, representing 17% of all PCORI CER awards

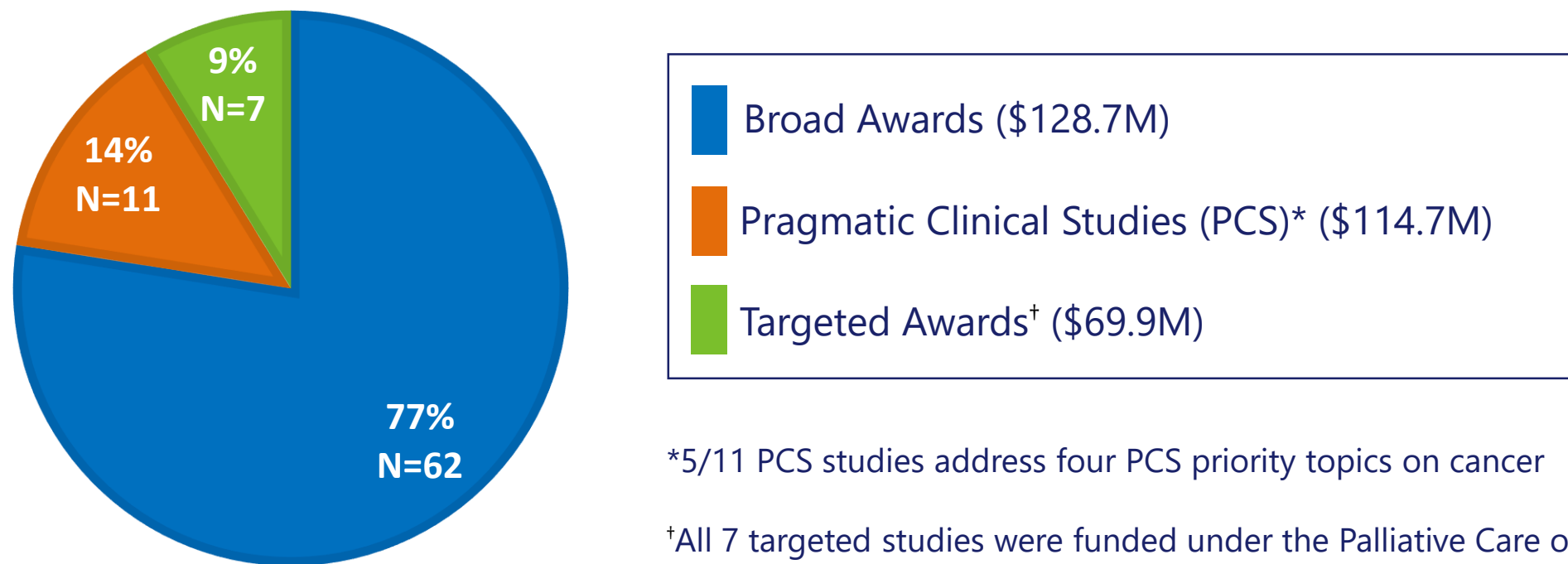
In addition, PCORI has also awarded:

- 45 Engagement Awards: \$8.6M
- 3 Dissemination & Implementation Awards: \$4.7M
- 1 PCORnet Rapid Research Award: \$860,000
- 2 Systematic Reviews: \$830,000

PCORI's Cancer Research Portfolio by Award Type



PCORI's Cancer Research Portfolio by Award Type (N=80 studies)

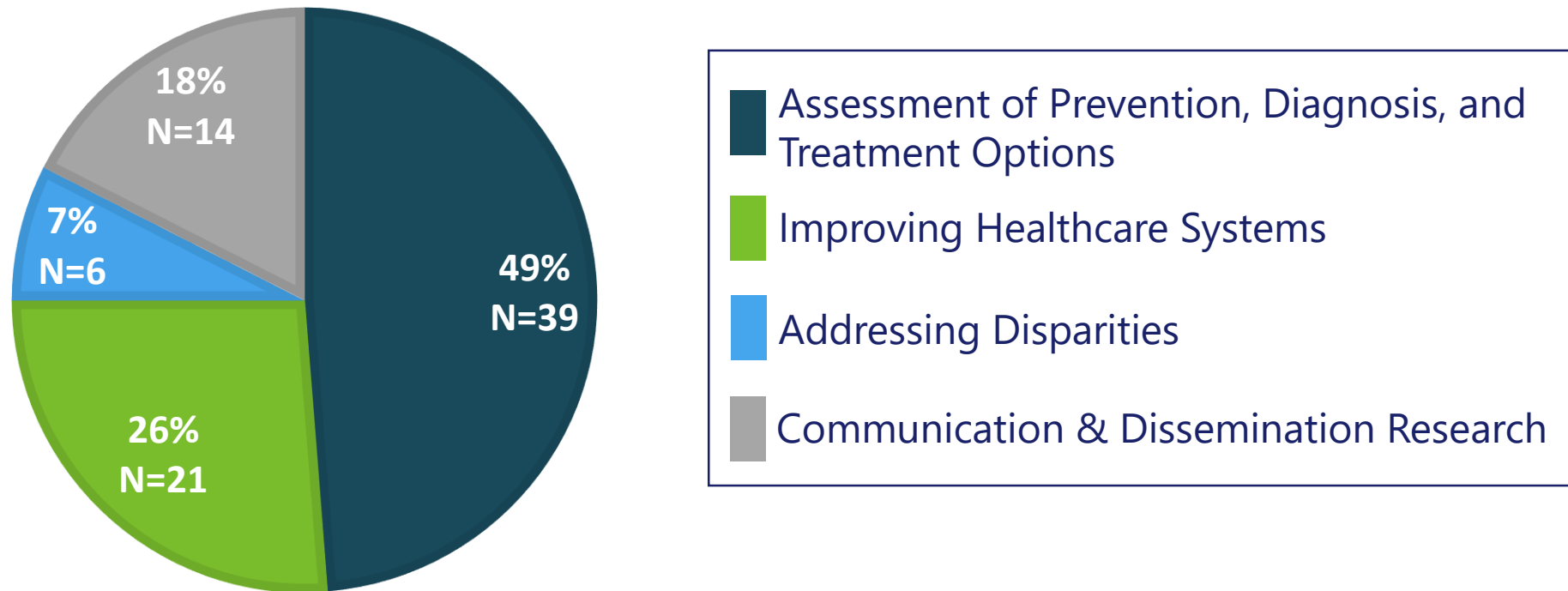


*5/11 PCS studies address four PCS priority topics on cancer

†All 7 targeted studies were funded under the Palliative Care or Symptom Management targeted PFAs, which were not focused exclusively on cancer

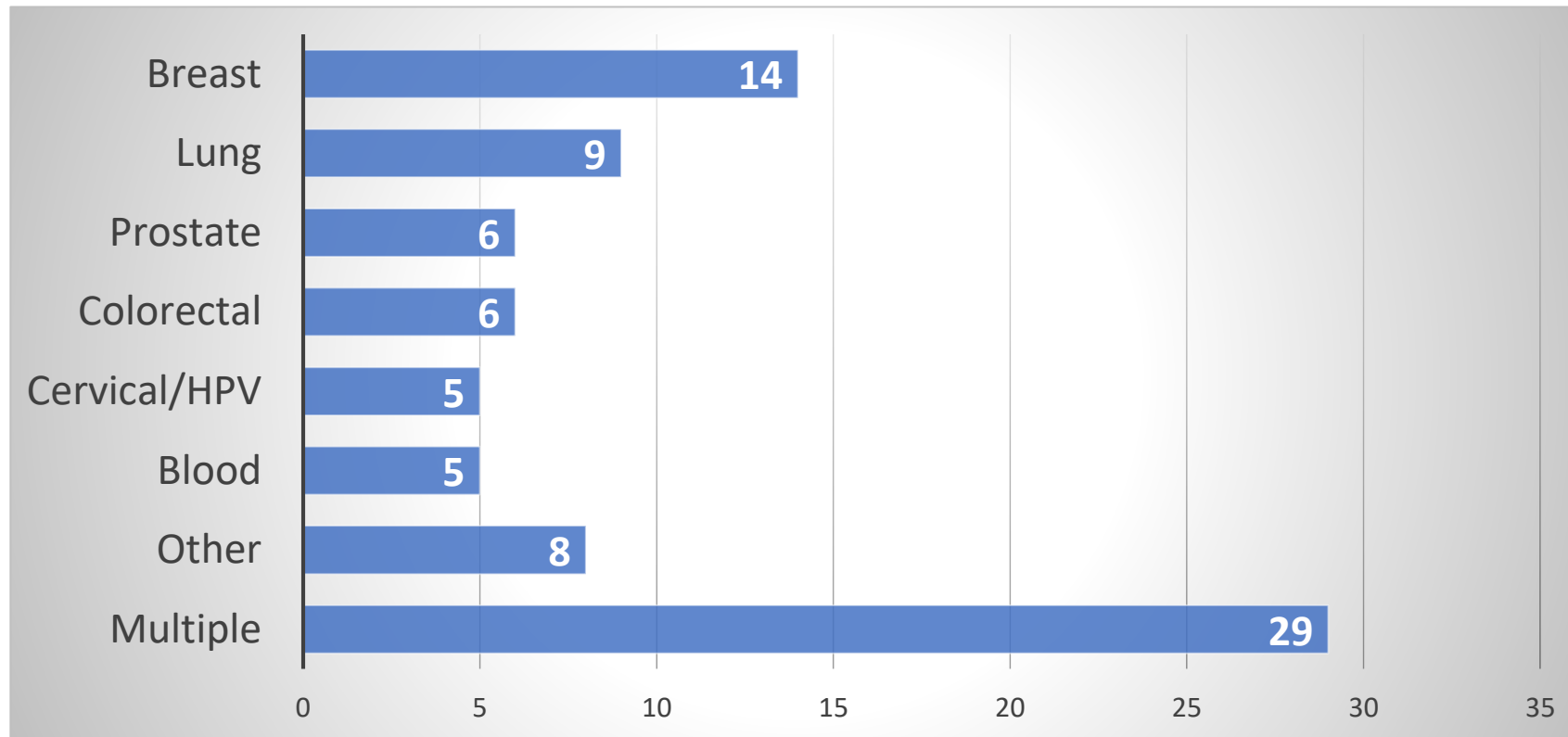
The Cancer Research Portfolio Spans PCORI's National Priority Areas

**PCORI's Cancer Research Portfolio by National Priority Area
(N=80 studies)**



The Research Portfolio Includes a Range of Cancer Types

Number of Research Studies by Cancer Type*



*Categories are not mutually exclusive

Key Portfolio Characteristics

- 70% (N=56) are randomized controlled trials
 - Of these, 87.5% (N=49) enroll patients from multiple sites
 - 57% of RCTs (N=32) include large patient samples (≥ 500 participants)
- 25% (N=20) of all cancer studies enroll caregivers
- Stakeholders are significantly engaged in the research process
 - Planning the study, finalizing interventions (81%)
 - Recruitment and retention of study participants (83%)
 - Data analysis and/or review of results (69%)
- Over 50% of studies have a patient or stakeholder co-investigator

The Cancer Research Portfolio Includes a Range of Interventions

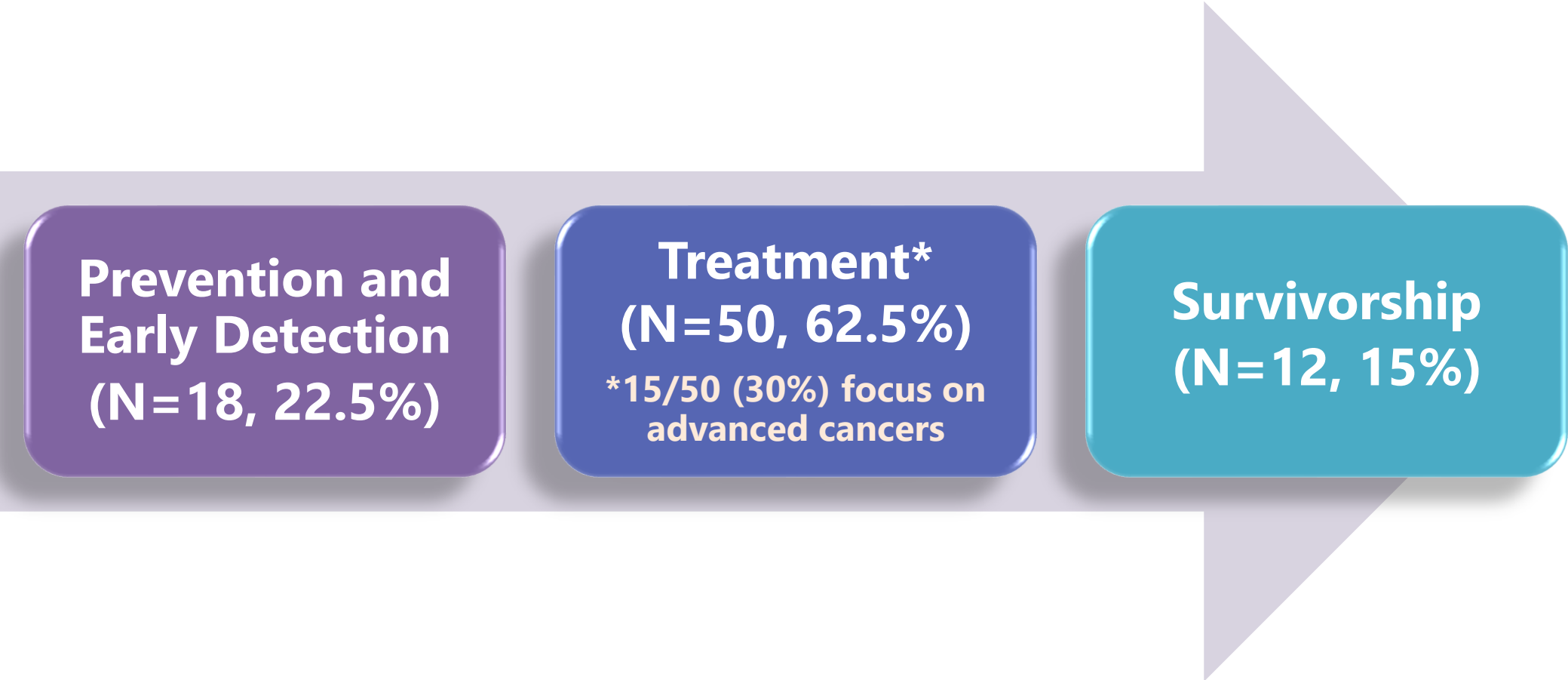
Clinical Interventions

- Primary Treatment Modalities (n=15)
 - Surgery, Radiation, Drugs
- Screening & Surveillance Protocols (n=8)
- Behavioral & Psychological (n=5)
- Complementary & Alternative Medicine (n=4)

Care Delivery/ Communication Strategies

- Models of Care (n=21)
- Decision Support Tools (n=16)
- Education & Communication (n=14)
- Telemedicine/mHealth (n=8)
- Patient Navigation (n=5)

Research Studies Address Decisional Dilemmas Across the Patient's Cancer Journey



**Prevention and
Early Detection
(N=18, 22.5%)**

Treatment*
(N=50, 62.5%)
*15/50 (30%) focus on
advanced cancers

**Survivorship
(N=12, 15%)**

Research Studies Address Decisional Dilemmas Across the Patient's Cancer Journey

Prevention and Early Detection

- **Uptake in priority populations**
- Risk-based approaches to screening
- Communication and decision-making

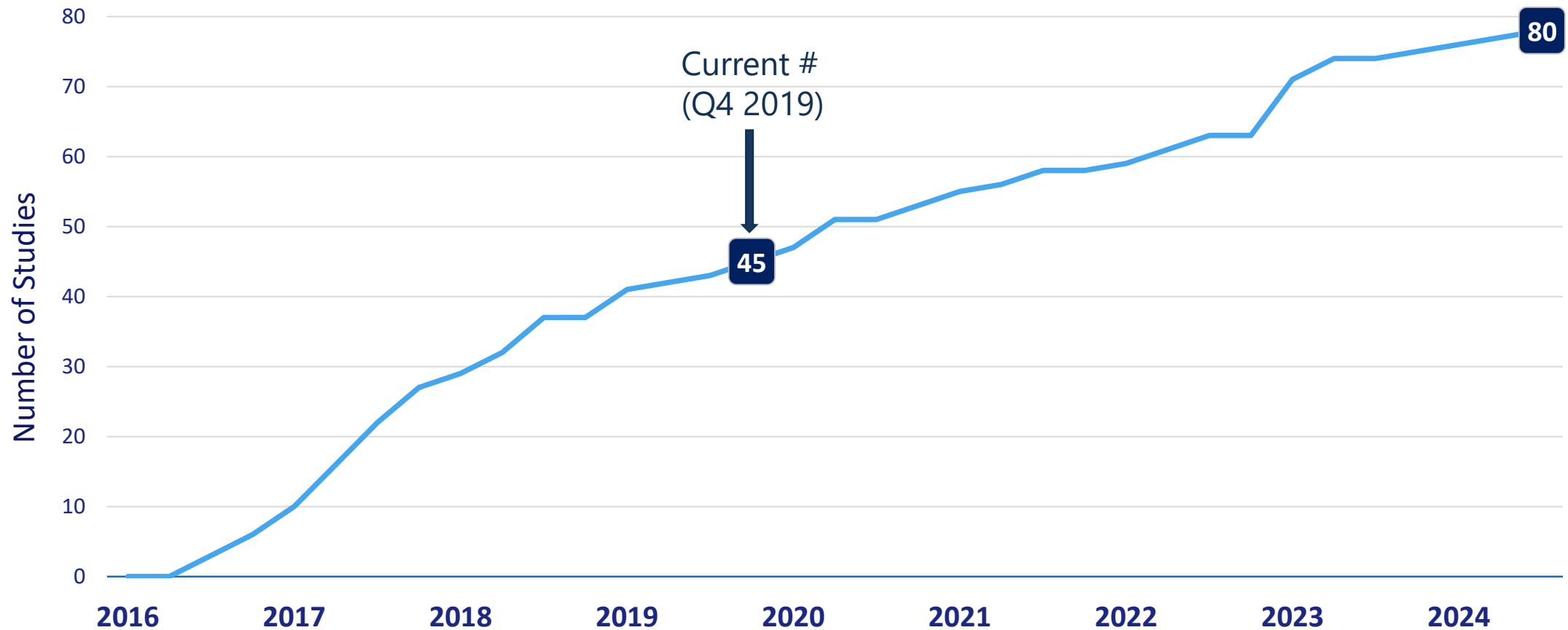
Treatment

- **Comparison of treatment options**
- Symptoms and side effects
- Caregiver burden
- Models of care
- Communication and decision-making

Survivorship

- **Post-treatment surveillance**
- Symptoms and side effects
- Caregiver burden
- Models of care

When Will Results from PCORI-Funded Cancer Studies Be Available?



PCORI's Cancer CER Portfolio

Study Spotlights Across the
Patient Journey

Prevention and Early Detection: Increase Uptake in Priority Populations



Increasing Colorectal Cancer Screening Among Hispanic Primary Care Patients. Ronald E. Myers, PhD (Principal Investigator), Thomas Jefferson University

Tailored Decision and Navigation Support Can Address Colorectal Screening Disparities

Ronald E. Myers, PhD, Thomas Jefferson University



Research Question	Does active decision support with a patient navigator improve colorectal cancer screening rates among Hispanic patients compared to mailed educational information?
What's New	Tailoring of decision and navigation support incorporating preferences for screening among Hispanic patients
Design	Randomized control trial at 5 primary care practices; N=400
Key Findings	Telephonic decision and navigation support increased colorectal screening (78%) compared to mailed information (44%).
References	Myers RE, Stello B, Daskalakis C, et al. Decision Support and Navigation to Increase Colorectal Cancer Screening Among Hispanic Patients. Cancer Epidemiol Biomarkers Prev. 2018 Oct 17. doi: 10.1158/1055-9965.EPI-18-0260. Myers RE, DiCarlo M, Romney M et al. Using a health system learning community strategy to address cancer disparities. Learn Health Sys. 2018;e10067. DOI: 10.1002/lrh2.10067

Engagement Enhances Intervention



"Members reviewed ... materials in both English and Spanish, helped to **frame terminology in plain language that was culturally sensitive and literacy appropriate**.....[They] recommended making **financial counseling** support available to those individuals in need of such services in order to undergo screening and follow-up as needed. Importantly, this **service was routinely made available** to study participants as part of a pre-colonoscopy visit."

– Dr. Myers

Thomas Jefferson University

Quote from the Project Monitoring Report

Treatment: Compare Different Clinical Options



- **Generating Critical Patient-Centered Information for Decision Making in Localized Prostate Cancer.** David Penson, MD, MPH (Principal Investigator), Vanderbilt University
- **Contralateral Prophylactic Mastectomy and Breast Cancer: Clinical and Psychosocial Outcomes.** Abenaa Brewster, MD (Principal Investigator), The University of Texas MD Anderson Cancer Center

Long-Term Side Effects Remain After Treatment for Early Stage Disease

David Penson, MD, MPH, Vanderbilt University

Research Question	What are the side effects of treatments (surgery, radiation, and active surveillance) at 3 years for localized prostate cancer?
What's New	Comparison of modern prostate cancer treatments with active surveillance in a diverse population
Design	Prospective observational study; N=2550
Key Findings	• Men who had surgery reported a greater decline in sexual function and worse urinary incontinence compared to radiation or active surveillance at 3 years. • Men who had radiation reported worse bowel function at 6 months compared to surgery and active surveillance; no significant differences at 3 years.
Reference	Barocas DA, Alvarez J, Resnick MJ, et al. Association Between Radiation Therapy, Surgery, or Observation for Localized Prostate Cancer and Patient-Reported Outcomes After 3 Years. <i>JAMA</i> . 2017 Mar 21;317(11):1126-1140. doi: 10.1001/jama.2017.1704. PubMed PMID: 28324093.

Dissemination Activities Extend Reach of Results

- PCORI funded a Dissemination and Implementation award to update an existing prostate cancer treatment decision aid with more representative data generated by this PCORI-funded study
- PCORI co-hosted a Congressional briefing with Men's Health Network on shared decision making for prostate cancer. Dr. Penson and a patient partner presented findings from their study
- Online Continuing Medical Education/Continuing Education activity highlighted findings from this study and another PCORI-funded study about contemporary treatments for localized prostate cancer

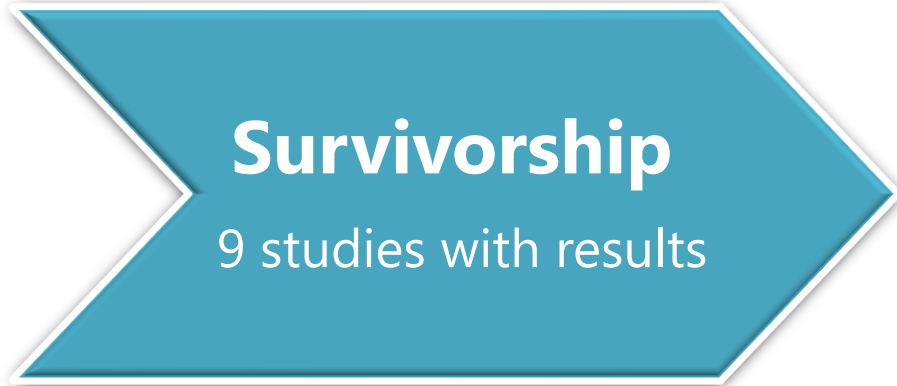
Mental and Social Well-Being Impacts Breast Cancer Treatment Choice and Adjustment Post-Treatment

Abenaa Brewster, MD, University of Texas MD Anderson Cancer Center



Research Question	How is a woman's mental and social well-being affected by the choice to remove both breasts (cancer and healthy) versus remove part or all of the breast with cancer but keep the healthy breast?
What's New	Assessment of well-being after surgery among women with sporadic breast cancer
Design	Prospective observational study; N=288
Key Findings	Compared with women who kept the healthy breast, women who had both breasts removed (17%) had: • More cancer worry pre-surgery • More concerns about body image after surgery • The same amount of worry about their cancer after surgery • The same quality of life before surgery • A lower quality of life after surgery
Reference	Parker PA, Peterson SK, Shen Y, et al. Prospective Study of Psychosocial Outcomes of Having Contralateral Prophylactic Mastectomy Among Women With Nonhereditary Breast Cancer. <i>J Clin Oncol.</i> 2018 Jul 25;JCO2018786442. doi: 10.1200/JCO.2018.78.6442. [Epub ahead of print] PubMed PMID: 30044695.

Survivorship: Optimize Post-Treatment Surveillance

A large, teal-colored arrow pointing to the right, with a white outline and a slight drop shadow. It is centered on the slide.

Survivorship

9 studies with results

Improving the Effectiveness of Routine Surveillance Following Lung Cancer Resection. Benjamin David Kozower, MD, MPH (Principal Investigator), The Alliance for Clinical Trials in Oncology Foundation

More Frequent Surveillance Following Lung Cancer Resection Does Not Improve Survival

Benjamin David Kozower, MD, MPH, The Alliance for Clinical Trials in Oncology Foundation



Research Question	Does intensity of surveillance post-treatment improve survival?
What's New	Evaluate question in a large U.S. population outside clinical trial context
Design	Retrospective observational cohort, N=4463
Key Findings	Compared with getting an imaging test every 12 months, receiving tests at 3- or 6-month intervals made no difference in: • How soon doctors detected a recurrence • How likely patients were to live five years after their first surgery
Reference	McMurry TL, Stukenborg GJ, Kessler LG, et al. More Frequent Surveillance Following Lung Cancer Resection Is Not Associated With Improved Survival: A Nationally Representative Cohort Study. <i>Ann Surg.</i> 2018;268(4):632–639. doi:10.1097/SLA.0000000000002955

Engagement Influences Future Research



"This experience has dramatically impacted how to perform research of this type in the future. We recently submitted [an application for funding] to study the implementation of patient reported outcomes into the Society of Thoracic Surgeons [STS]. We spent a tremendous amount of time working with patients in the Alliance, STS leadership, and several STS taskforces to shape the project and support for the application. **Stakeholder engagement is critical!"**

– Dr. Kozower
The Alliance for Clinical Trials in Oncology Foundation

Ongoing Large Trials Will Provide Evidence to Inform Key Decisional Dilemmas



- **Prevention** - Comparative Effectiveness of System Interventions to Increase HPV Vaccine Receipt in Federally Qualified Health Centers (PI: Roshan Bastani, PhD, UCLA School of Public Health)
- **Screening** - A Preference-Tolerant RCT of Personalized vs. Annual Screening for Breast Cancer – The WISDOM Study (PI: Laura J. Esserman, MD, MBA, University of California, San Francisco)
- **Treatment** - CISTO: Comparison of Intravesical Therapy and Surgery as Treatment Options for Bladder Cancer (PI: John Gore, MD, MS, University of Washington)
- **Symptoms/side-effects management** - A Pragmatic Trial to Improve Colony Stimulating Factor Use in Cancer (PI: Scott D. Ramsey, MD, PhD, Fred Hutchinson Cancer Research Center)
- **Advanced Illness Care** - Comparative Effectiveness of Early Integrated Telehealth Versus In-Person Palliative Care for Patients with Advanced Lung Cancer (PIs: Jennifer S. Temel, MD & Joseph Greer, PhD, MS, Massachusetts General Hospital)

Lessons Learned from Large Trials

- Extra time and resources are needed to:
 - Communicate equipoise and minimize provider bias
 - Integrate system interventions into routine clinical workflow
- Significant coordination and relationship-building between awardee team and study sites is critical
 - Site-specific recruitment tracking is essential to understand capacity and guide targeted enrollment strategies
 - Awardee team site visits and regular calls with study teams at individual sites
 - Regular cross-site communication and sharing opportunities
- Systematic monitoring of intervention implementation processes across sites is needed to ensure fidelity

Summary

- PCORI awardees engage with stakeholders to address important decisional dilemmas across the patient's cancer journey
- Studies emphasize the full spectrum of outcomes that matter to patients and caregivers
- Many studies build on prior trials, taking projects to scale in real-world settings and with patients who are often excluded from traditional trials
- When possible, PCORI awardees leverage existing registries and clinical trials infrastructure to address questions of importance to stakeholders

Summary

- Conducting large, multi-site trials in real-world practice settings requires significant planning, time, and resources
 - Consider planning/pilot phase to ascertain feasibility
- Many therapeutic trials are likely to require follow-up beyond the 5-year funding period to assess long-term clinical outcomes
 - Consider partnership with other funders or supplemental PCORI funding for longer term follow-up

Current Initiatives and Future Considerations



- Current research areas of interest in Cycle 3 2019 Broad PFA
 - Dosing of anti-neoplastic agents in adults
 - Genetic sequencing to guide cancer treatment
- Additional areas for future CER consideration
 - Diagnostic interventions
 - Novel treatments (targeted or immunotherapies) in real-world settings
 - Issues specific to post-treatment cancer survivors and caregivers
 - Issues specific to older cancer patients with multiple chronic conditions
 - Cancers with increasing incidence among young adults (Sung H et al., Lancet Public Health 2019)

Questions Discussion Guidance

Public Comment Period

Kristin Carman, MA, PhD

Director, Public and Patient Engagement

Wrap Up and Adjournment

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