# Advisory Panel on Rare Disease (RDAP) Virtual Meeting: Winter 2021

December 13, 2021

**Mat Edick, PhD**
Chair, RDAP

**Doug Lindsay, BS**
Co-Chair, RDAP

RDAP PCORI Staff Team

- Carly Khan, PhD, MPH, RN
- Nora McGhee, PhD
- Fatou Ceesay, MPH
- Meghan Berman, MPH
- Rohini Mohanraj, MHA

pcori®
PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

# RDAP Chairs

**Mat Edick, PhD**
Chair, Advisory Panel on Rare Disease
Director of the Center for Strategic Health Partnerships, Michigan Public Health Institute

**Doug Lindsay, BS**
Co-Chair, Advisory Panel on Rare Disease
Personal Medical Consultant and Founder of Doug Says LLC.

# Housekeeping

- Please note that today's webinar is being recorded for posting on PCORI's website.
- Members of the public are invited to listen to the teleconference and view the webinar.
- Meeting materials can be found on the PCORI website. The recording of the webinar will also be made available to the public after this event.
- Anyone may submit a comment through the webinar chat function.
  - No public comment period is scheduled

Please visit www.pcori.org/events for more information.

# COI Statement

Welcome to the Rare Disease Advisory Panel Winter 2021 virtual meeting.

I want to remind everyone that disclosures of conflicts of interest of members of the Advisory Panel are publicly available on PCORI's website.  Members of the Rare Disease Advisory Panel are reminded to update your conflict-of-interest disclosures if the information has changed, in addition to completing your annual disclosure. You can do this by contacting your staff representative, Rohini Mohanraj.

Finally, if the Rare Disease Advisory Panel will deliberate or act on a matter that presents a conflict of interest for you, please inform one of the co-chairs so we can discuss how to best address the issue.

# Day 1 Meeting Agenda

| Start Time | Agenda Items | Presenters & Discussion Facilitator |
|---|---|---|
| 1:00 PM | Welcome, Introductions, and Setting the Stage | Mat Edick, Doug Lindsay |
| 1:30 PM | Introduction of RDAP PCORI staff & RDAP Program Staff Update | Carly Khan |
| 1:40 PM | RDAP Speakers: Experience with the Patient and Caregiver Journey from Diagnosis to Treatment and Beyond | Sarah Bacon, Danielle Boyce, Doug Lindsay |
| 2:25 PM | Discussion: PCORI-funded Rare Disease Portfolio Update | Carly Khan, Nora McGhee, Rohini Mohanraj, Doug Lindsay |
| 3:25 PM | BREAK (20 minutes) | |
| 3:45 PM | Strategic Planning: Proposed Research Agenda | Marina Broitman, Mat Edick |
| 4:15 PM | Round Robin Brainstorming Session | Mat Edick |
| 4:45 PM | Acknowledgments and Recap | Mat Edick, Doug Lindsay |
| 5:00 PM | Adjourn | |

# RDAP Panelist Introductions

# RDAP Members

## CLINICIANS

Natario Couser (Virginia Commonwealth University)

Nancy Rose (American College of Medical Genetics and Genomics)

Laura Tosi (Children's National Hospital)

## RESEARCHERS

Heather Adams (University of Rochester Medical Center)

Sonia Jain (University of California, San Diego)

Jasvinder Singh (University of Alabama at Birmingham)

## POLICY MAKERS

Saira Sultan (Connect4Strategies)

## INDUSTRY

Salman Hussain (Charles River Associates)

Giovanna Devercelli (Takeda Pharmaceuticals)

## PATIENTS, CAREGIVERS, AND PATIENT ADVOCATES

Mat Edick (Chair)

Doug Lindsay (Co-Chair)

Sarah Bacon (Patient, advocate, and writer)

Danielle Boyce (COPD Foundation)

Deanna Fournier (Histiocytosis Foundation)

Mileva Repasky (Castleman Disease Collaborative Network)

Bridget Reynolds (Patient and advocate)

## EX-OFFICIO MEMBER

Naomi Aronson (BCBSA)

# RDAP Staff Introductions

# PCORI Staff Team

**Carly Khan, PhD, MPH, RN**
Associate Director,
*Healthcare Delivery and Disparities Research*

**Nora McGhee, PhD**
Senior Program Officer,
*Clinical Effectiveness and Decision Science*

**Fatou Ceesay, MPH**
Program Officer,
*Engagement Awards*

**Meghan Berman, MPH**
Program Associate II,
*Public and Patient Engagement*

**Rohini Mohanraj, MHA**
Program Associate,
*Research Infrastructure*

# Program Updates

- Most rare disease comparative effectiveness research studies awarded through the Conducting Rare Disease Research using PCORnet® targeted funding announcement have kicked off and are getting started.
- Three new Engagement Awards on rare diseases have been funded.
  - Two are from Osteogenesis Imperfecta
  - Capacity building project for the rare disease community
- PCORI is accepting applications and nominations for new advisory panel members for 2022. All applications and nominations are due Thursday, March 31, 2022, by 5:00 pm EST. More information available here.
- Save the Date: The 2022 PCORI Annual Meeting will be held Wednesday, October 26 – Friday, October 28, 2022.

# RDAP Speakers: Experience with the Patient and Caregiver Journey

## Diagnosis to Treatment and Beyond

Sarah Bacon, MS
Danielle Boyce, MPH

pcori
PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

FROM ULTRA RARE DISEASE DIAGNOSIS TO CITIZEN SCIENTIST

SARAH H. BACON, RDAP PANELIST

# LIVING WITH ZEBRAS

# OVERVIEW

- DIAGNOSIS WITH A ZEBRA, A RARE OR ULTRA RARE DISEASE
- FINDING THE RIGHT CLINICIAN ALLY
- POLITICS
- PATIENT ADVOCACY & CITIZEN SCIENCE

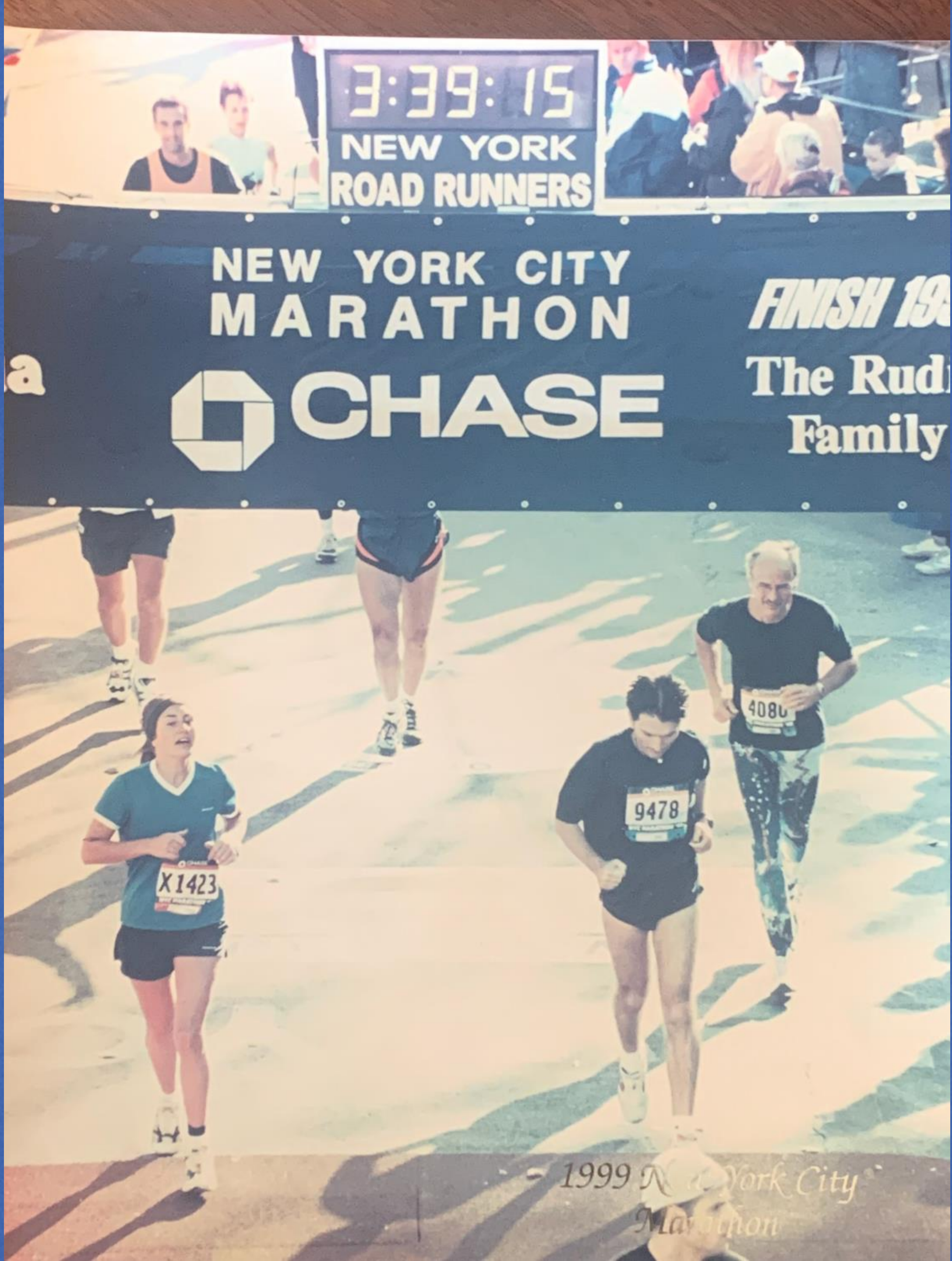
# CANARY IN THE COAL MINE

LIFE LONG ATHLETE .

FIRST LUNG COLLAPSE AT AGE 33 MISDIAGNOSED AS PLEURISY.

SUBSEQUENTLY DEVELOPED SUDDEN ONSET ASTHMA; PRESCRIBED STEROID INHALERS.

PCP GAVE ME THE GREEN LIGHT FOR HORMONE INTENSIVE EGG HARVESTING PROTOCOL IN 2010 DESPITE A 2005 RADIOLOGIST REPORT DIAGNOSING ME WITH LYMPHANGIOLEIOMYOMATOSIS, AN ESTROGEN SENSITIVE DISEASE.



# DIAGNOSIS

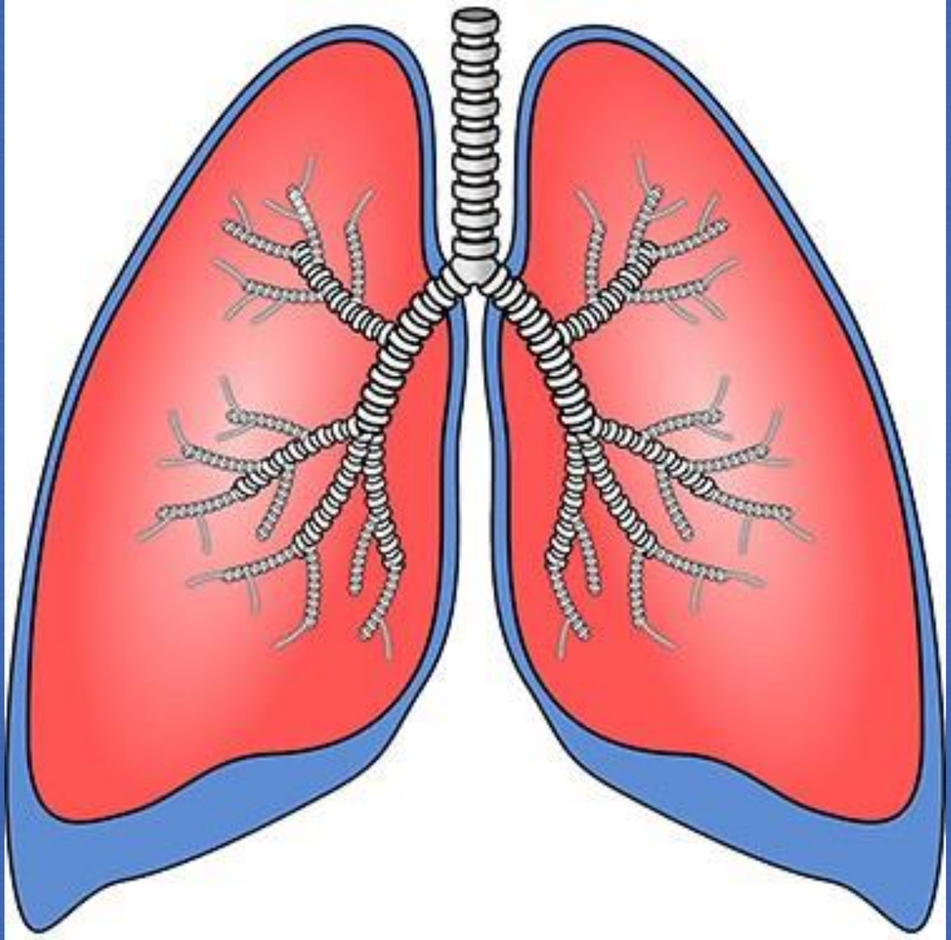
BOUTS OF BRONCHITIS INCREASED IN SEVERITY AND DURATION AFTER EGG HARVESTING.

WALKING UP STAIRS, SPRINTING ACROSS STREETS, CARRYING GROCERIES MADE ME BREATHLESS.

AFTER BURSTING AN EAR DRUM COUGHING, I WENT TO SEE MY PCP FOR A CHEST X-RAY, JANUARY 2013.

RADIOLOGY TECH SAID HE HADN'T "SEEN THIS SINCE MEDICAL SCHOOL".

PULMONOLOGIST REVIEWED ALL CHEST IMAGERY FROM
MY PCP AND ASKED WHY I'D NEVER TOLD HIM I HAD LAM?
I'D NEVER HEARD OF IT.



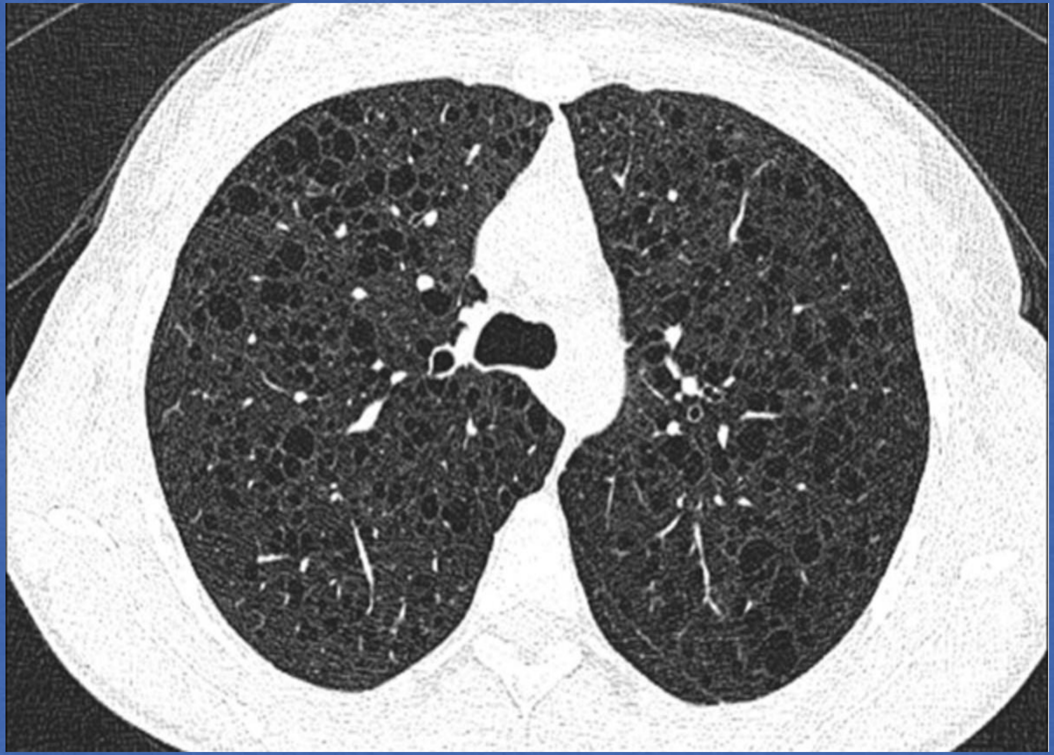
# LAM

LAM IS A PROGRESSIVE, ESTROGEN-SENSITIVE DISEASE FOUND IN WOMEN.

SMOOTH MUSCLE CELLS PROLIFERATE IN THE LUNGS AND ABDOMEN, CAUSING CYSTIC GROWTH. THOSE CYSTS SUPPLANT OXYGEN IN THE LUNGS, OFTEN LEADING TO DOUBLE LUNG TRANSPLANT. CYSTS IN THE ABDOMEN CAUSE HARM WHEN ATTACHED TO THE KIDNEY OR UPON BURSTING.

FDA APPROVED IMMUNOSUPPRESSANT RAPAMYCIN IN 2011 TO SLOW CYSTIC GROWTH IN LAM PATIENTS. LAM IS AMONG THE FIVE PERCENT OF RARE DISEASES WHICH HAVE ANY TREATMENT.

WITH A SURVIVAL RATE OF FIFTY PERCENT FIVE YEARS OUT FROM A DOUBLE LUNG TRANSPLANT, SLOWING CYSTIC GROWTH ALONE WASN'T GOOD ENOUGH FOR ME.



# THE RABBIT HOLE

MY GOAL WAS TO MAINTAIN MY ACTIVE, ENGAGED LIFESTYLE.

I SET OUT TO FIND LAM SPECIALIST CLINICIAN/INVESTIGATOR AT A TEACHING UNIVERSITY TO ADVANCE RESEARCH.

TWO ESTABLISHED PATIENT ADVOCACY GROUPS IN THE U.S. DEDICATED TO PROMOTING LAM RESEARCH REFERRED ME TO SPECIALISTS.

COLUMBIA UNIVERSITY'S LAM LAB WAS THE FIRST LAM CLINIC IN THE COUNTRY, HAD THE LARGEST PATIENT ENROLLMENT OF ALL LAM CLINICS, AND THOUGHT OUTSIDE OF THE BOX.

COLUMBIA BECAME MY HORSE IN THE RESEARCH RACE.

# PARTNERS & ALLIES

DR. JEANINE D'ARMIENTO, FOUNDER OF THE LAM LAB, COLUMBIA UNIVERSITY, GAVE ME A TREATMENT PLAN.

HMGA2 PATHWAY

RESEARCH. MELANOMA

CONNECTION.

CONCRETE PROGRESS DURING A BLEAK TIME & REVELATION ON EARLY STAGE RESEARCH'S AFFORDABILITY.

BECAME A STRATEGIC LAM LAB SUPPORTER.

Welcome to

The Center for LAM and Rare Lung Diseases at Columbia University

# ADVOCACY & CITIZEN SCIENCE

**DISCOVERING THE INEQUITIES RARE DISEASES FACE WITH REGARD TO RESEARCH FUNDING & 2013 SEQUESTER CUTS TO NIH BUDGET LEAD TO FIRST PATIENT ADVOCATE ARTICLE IN THE ATLANTIC, APRIL 2013.**

**SUBSEQUENT INVITATIONS TO SPEAK AND WRITE ABOUT RARE DISEASES FOR *THE WASHINGTON POST, FAST COMPANY, NEW YORK MAGAZINE*, & FORTHCOMING BOOK, *LIVING WITH ZEBRAS*.**

**LAMPOSIUM POLLS & MELANOMA DATA.**

**INTRODUCED DR. D'ARMIENTO TO THE CSO OF THE MELANOMA RESEARCH ALLIANCE ON PRELIMINARY MELANOMA/LAM DATA.**

**SUCCESSFULLY ADVOCATED NOVARTIS TO MAKE GOOD ON THEIR PROMISE OF AN IN KIND GLEEVEC DONATION TO THE LAM LAB FOR THE LAMP-1 TRIAL.**

*The Atlantic*

HEALTH

Medical Research Cuts Have Immediate Health Effects

The sequestration means many promising research trials will go unfunded. That immediately undermines a sense of hope for some patients with incurable conditions.

By Sarah Bacon

APRIL 17, 2013

# THE BIG PICTURE

TO UNDERSTAND THE LANDSCAPE I ATTENDED MANY RARE CONFERENCES, INCLUDING PARTNERING FOR CURES, NORD, DIA, GLOBAL GENES, RDLA & NATIONAL RARE DISEASE DAY AT THE NIH.

I ADVISED THE NCATS ON THEIR PATIENT TOOL KIT.

ADVISOR ON GLOBAL GENES GRANTEES.

URGED LAW MAKERS TO PASS THE OPEN ACT AND 21ST CENTURY CURES ON THE HILL DURING RARE DISEASE WEEK.

SOMETIMES WE GOT RESULTS AND SOMETIMES NOT, BUT PATIENT VOICES MUST ALWAYS BE AT THE TABLE.

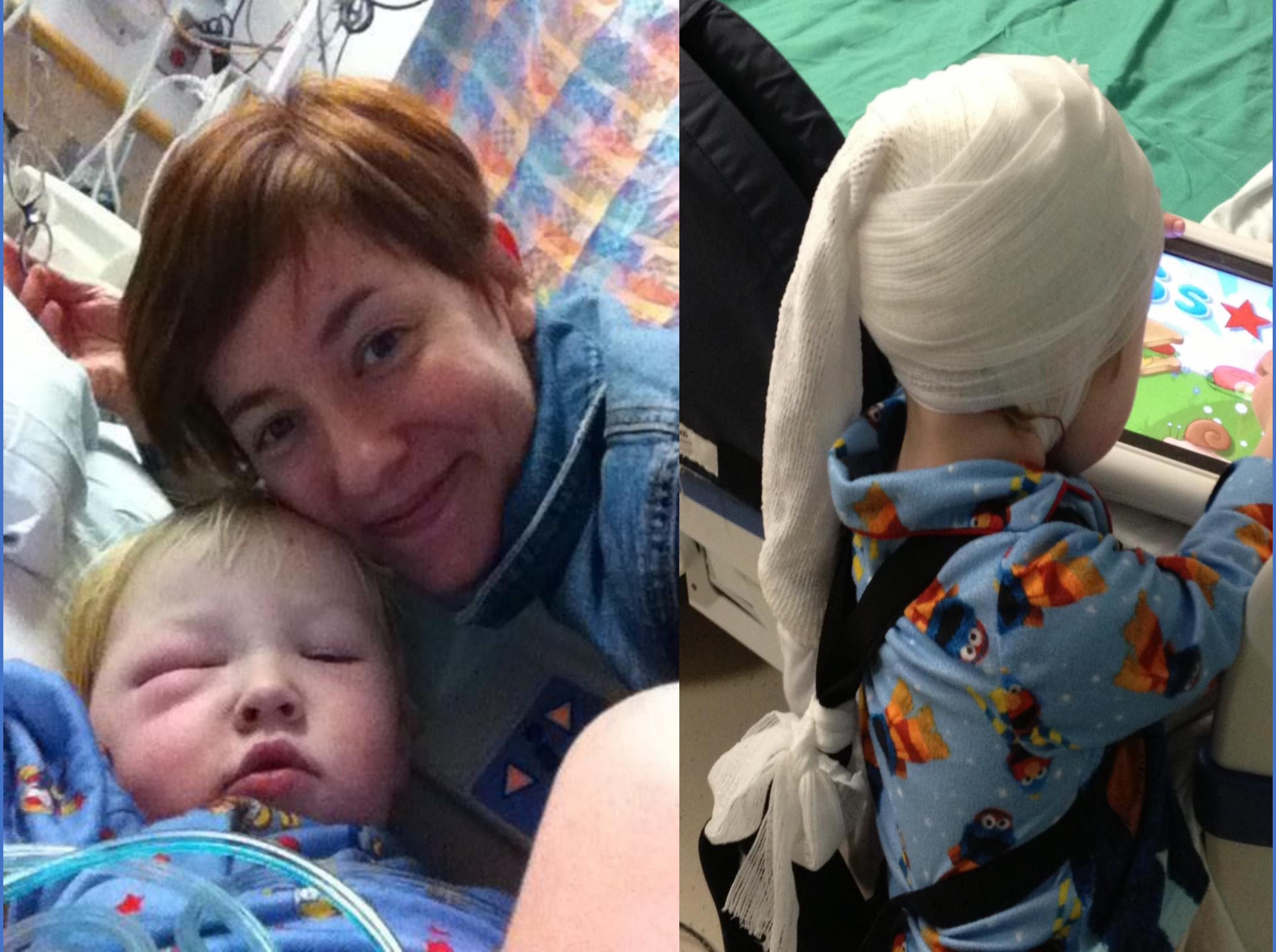
**Photo credits:**

**Cover and Closer: Andrew Boyers/Reuters c/o *The Guardian***
**Slide 3: Sarah Bacon**
**Slide 4: Edward-Elmhurst Health**
**Slide 5: Image courtesy of Joyce Lee, MD, MAS. c/o Merck Manuals**
**Slide 6: Sarah Bacon**
**Slide 7: The LAM Lab, Columbia University; Alpha One Foundation**
**Slide 8: Sarah Bacon**
**Slide 9: Sarah Bacon**

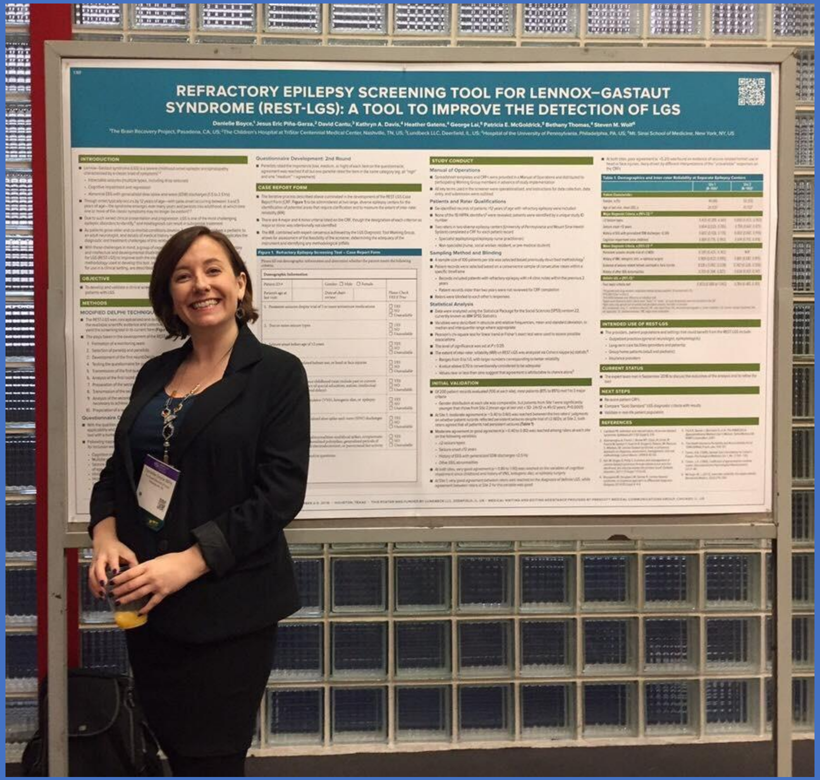
# THANK YOU.

# Charlie’s Rare and Important Story

Danielle Boyce, MPH, DPA
Charlie’s Mom



# The Making of an Advocate




INFANTILE SPASMS
Infantile Spasms
www.ISWEEK.org
FOX 7 STUDIO
PHILADELPHIA
FOX 7
7:31
41°

# Perspective: multi-stakeholder research

- Patients, caregivers, and advocacy group staff bring different perspectives
- Engage early, often, and meaningfully
- Consider our lifestyle and accommodate accordingly
- Don’t assume we won’t be interested in scientific programming
- Support the un- and under-diagnosed
- Develop PROs for people with intellectual and developmental disabilities
- Include caregiver & siblings when evaluating outcomes

# Questions

Thank you!
dboyce3@jhu.edu

# Discussion: PCORI-funded Rare Disease Portfolio Update

**Nora McGhee, PhD**
Senior Program Officer, Clinical Effectiveness and Decision Science

**Rohini Mohanraj, MHA**
Program Associate, Research Infrastructure

**Carly Khan, PhD, MPH, RN**
Associate Director, Healthcare Delivery and Disparities Research

**Precious Kolawole, BS**
Intern, Healthcare Delivery and Disparities Research

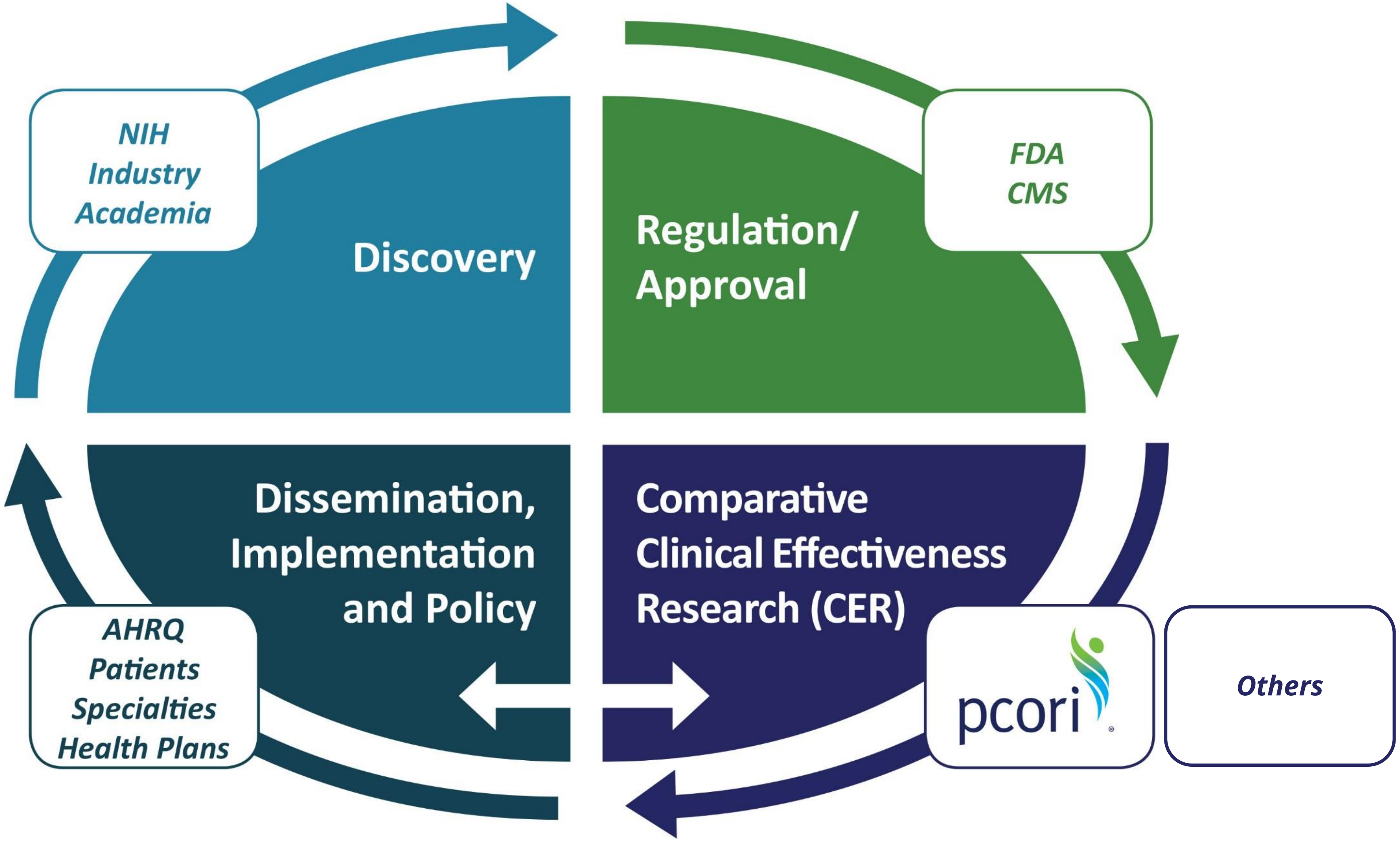
# Background

- PCORI utilizes NIH's definition of a rare disease, defined as a condition that affects **less than 200,000** in the United States.
- It is estimated that approximately **25-30 million** Americans have a rare disease (CDC).
- The exact cause of many rare diseases is still unknown and most rare diseases have no curative treatments.
- PCORI is committed to funding relevant research about what works best for individual patients.
    - This includes a commitment to funding research that addresses outstanding questions about the **prevention, diagnosis, and treatment of diseases and conditions, including rare diseases**.

# PCORI’s Research Portfolio

- Comparative Effectiveness Research (CER) compares two or more available healthcare options to determine what works best for patients.
- PCORI supports patient-centered outcomes research or PCOR, which is CER that focuses on traditional clinical outcomes as well as the needs, preferences, and outcomes most important to patients and caregivers.
- PCORI also supports Methods projects, which are studies to improve the methods available for patient-centered CER.
- As of September 2021, PCORI has awarded $2.4 Billion in CER and $155 Million towards Methods projects.

# Role of Comparative Clinical Effectiveness Research



*NIH*
*Industry*
*Academia*

Discovery

Regulation/
Approval

*FDA*
*CMS*

Dissemination,
Implementation
and Policy

Comparative
Clinical Effectiveness
Research (CER)

*AHRQ*
*Patients*
*Specialties*
*Health Plans*

*Others*

# PCORI's Investment in Research Studies on Rare Disease

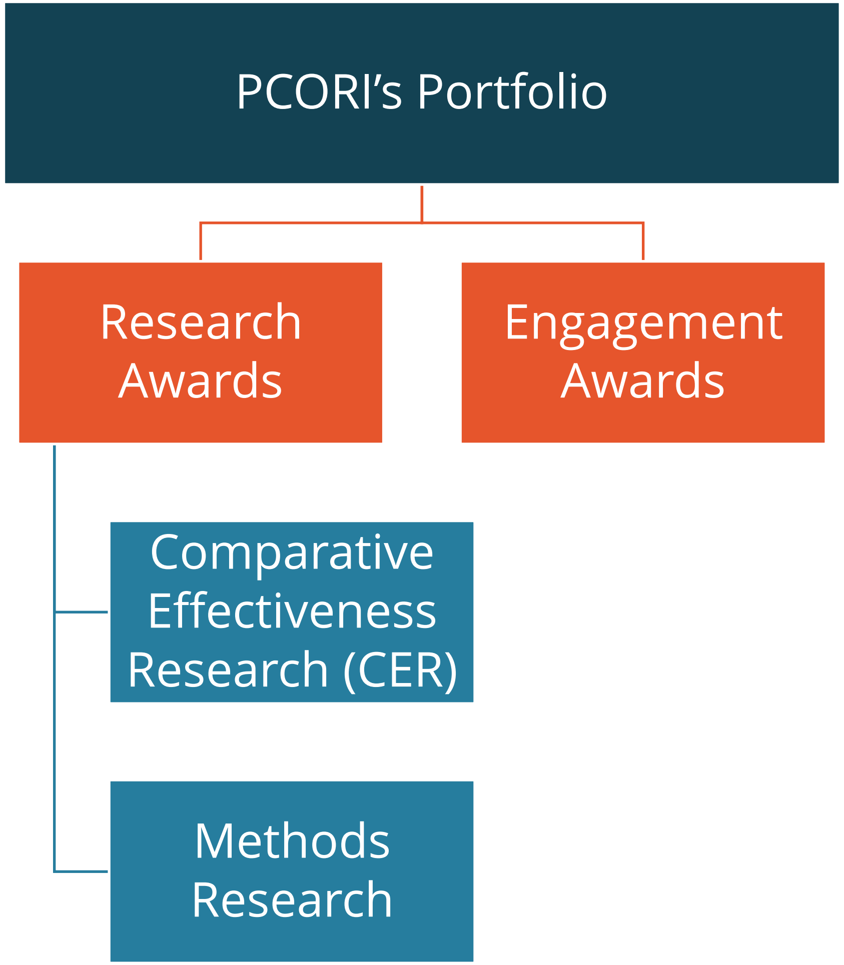
**PCORI HAS AWARDED**

**$112** MILLION TO FUND **38**

**COMPARATIVE CLINICAL EFFECTIVENESS RESEARCH STUDIES AND METHODS PROJECTS RELATED TO RARE DISEASE**

*As of November 2021*

# Rare Disease in PCORI's Research Portfolio



PCORI's Portfolio

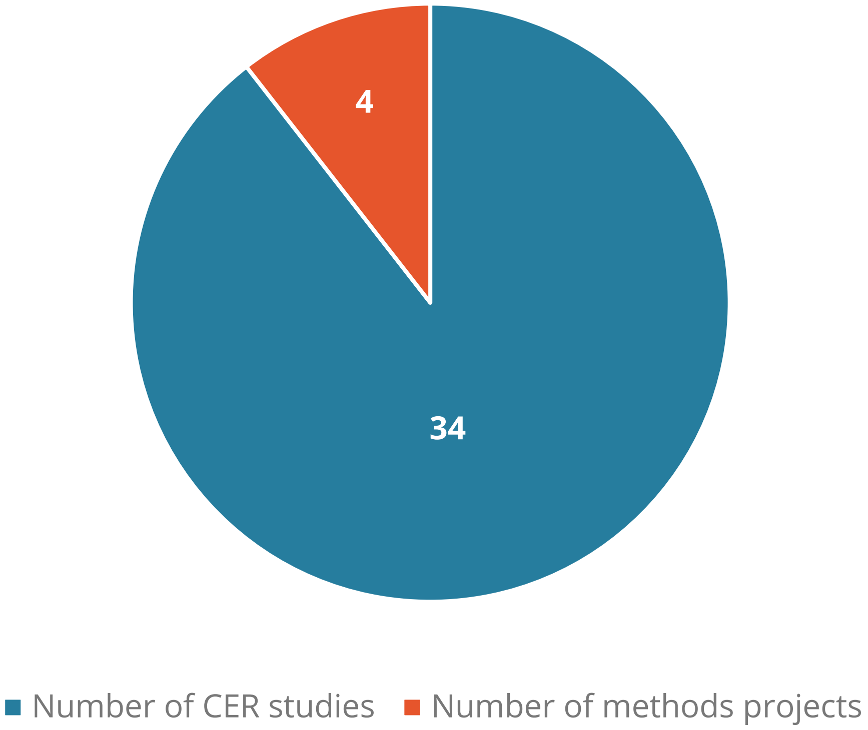
Research Awards

Engagement Awards

Comparative Effectiveness Research (CER)

Methods Research

## Number of funded Rare Disease Research Projects



# Rare Disease Methods Projects

- Methods projects address important methodological gaps and lead to improvements in the strength and quality of evidence generated by PCOR/CER studies.
- Projects involved Methodical Improvements for:
  - Small n SMART trials in the setting of Rare Diseases
  - Engaging Patients and Caregivers managing Rare Diseases in Clinical Guideline Development
  - Distributed Learning Framework for Integrating Evidence in Clinical Research Networks
  - Identification of Patients with Rare or Complex Diseases

Rare Disease Methods Projects

$3.8 M in funding

4 Awards

1 Project leveraging PCORnet resources

3 Completed Projects with Results

# Rare Disease CER Projects

- 29 studies are investigator-initiated research awards
- 5 studies were awarded through targeted PFAs.
- The Rare Disease CER Projects belong to the **4** remaining PCORI Priority Areas
  - Addressing Disparities (n=3)
  - Assessment of Prevention, Diagnosis, and Treatment Options (n=26)
  - Communication and Dissemination Research (n=2)
  - Improving Healthcare Systems (n=3)

Rare Disease CER Projects

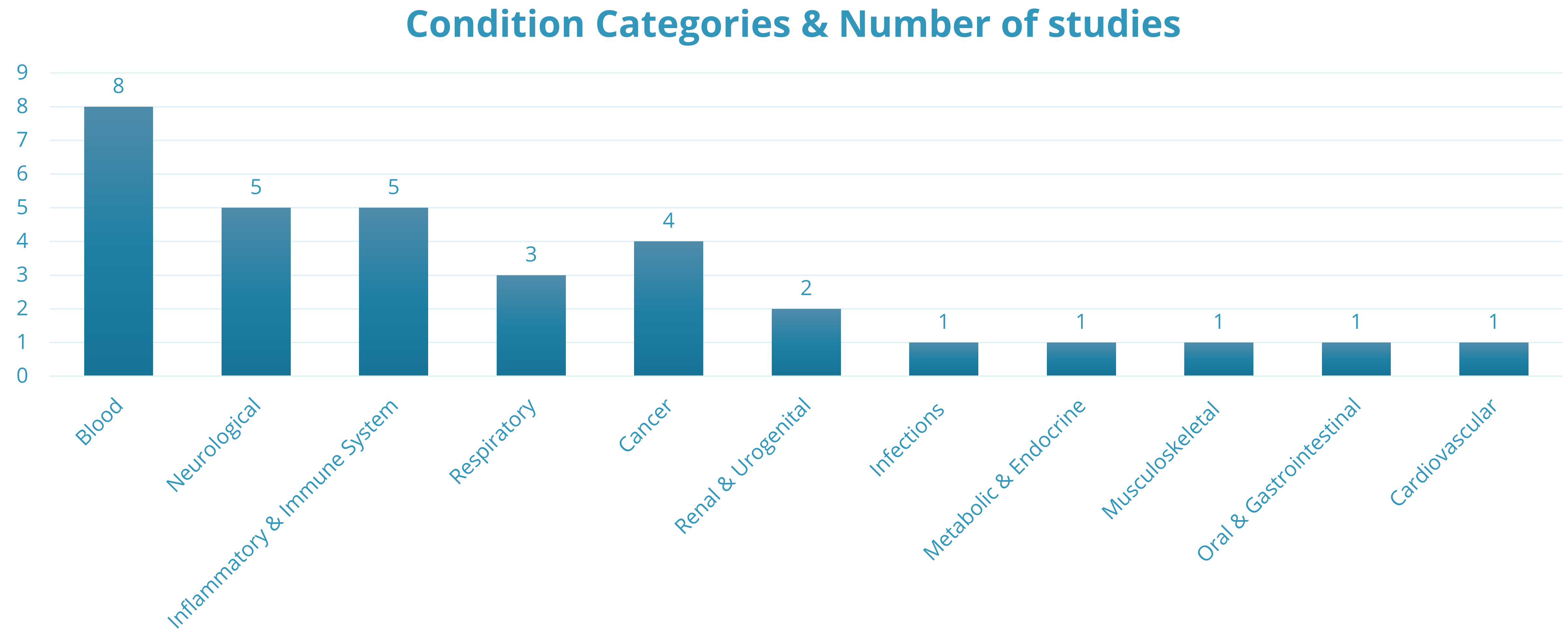
$108 M in funding

34 Awards

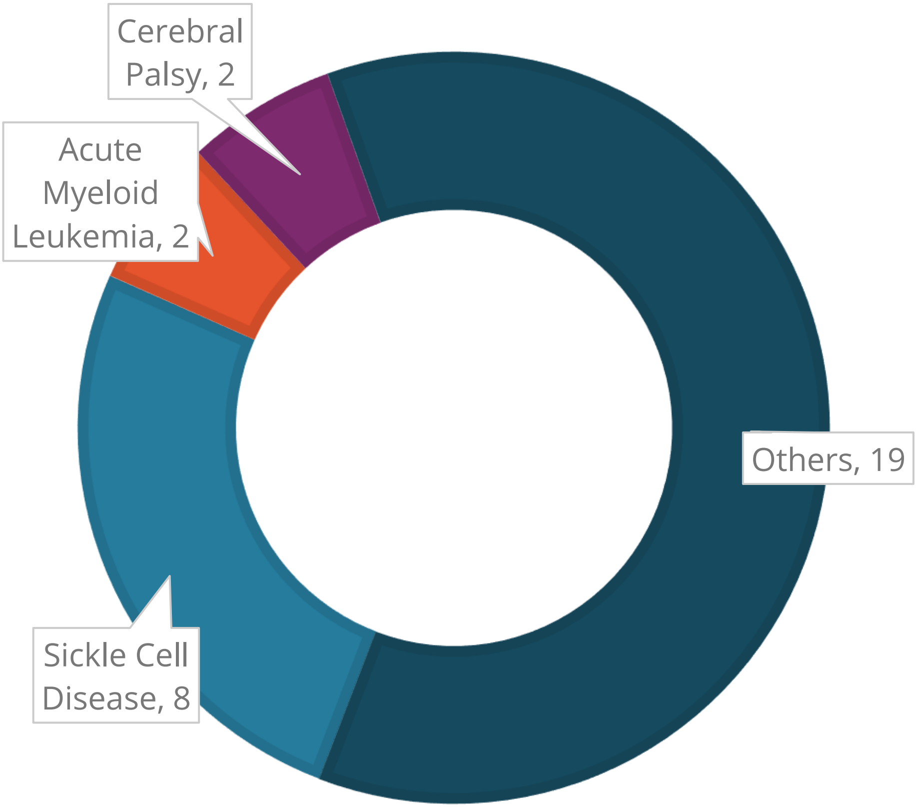
6 Projects leveraging PCORnet resources

19 Completed Projects with Results

# Primary Rare Disease Categories studied through CER



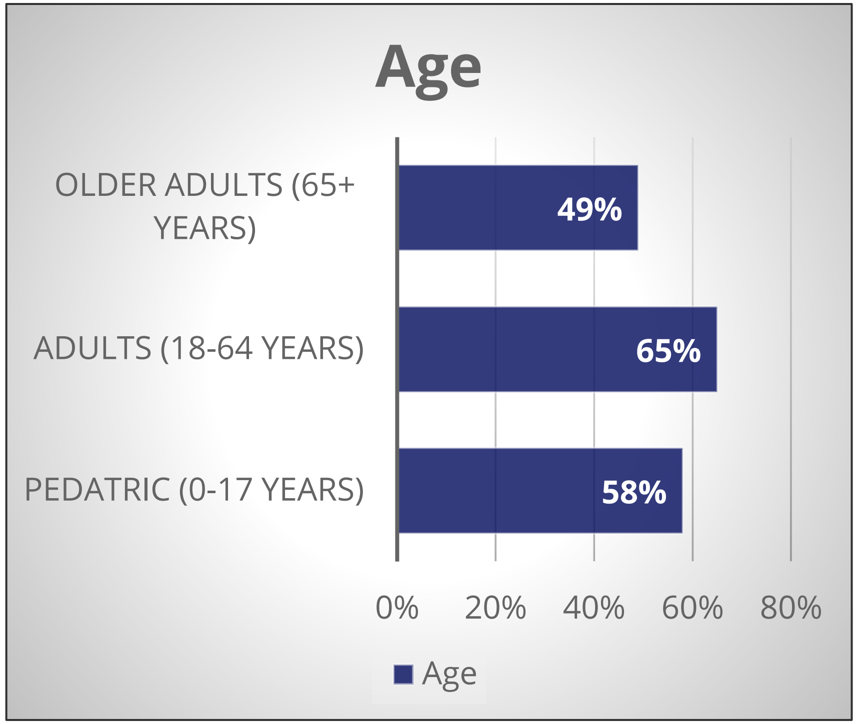
# Rare Diseases in PCORI CER Portfolio

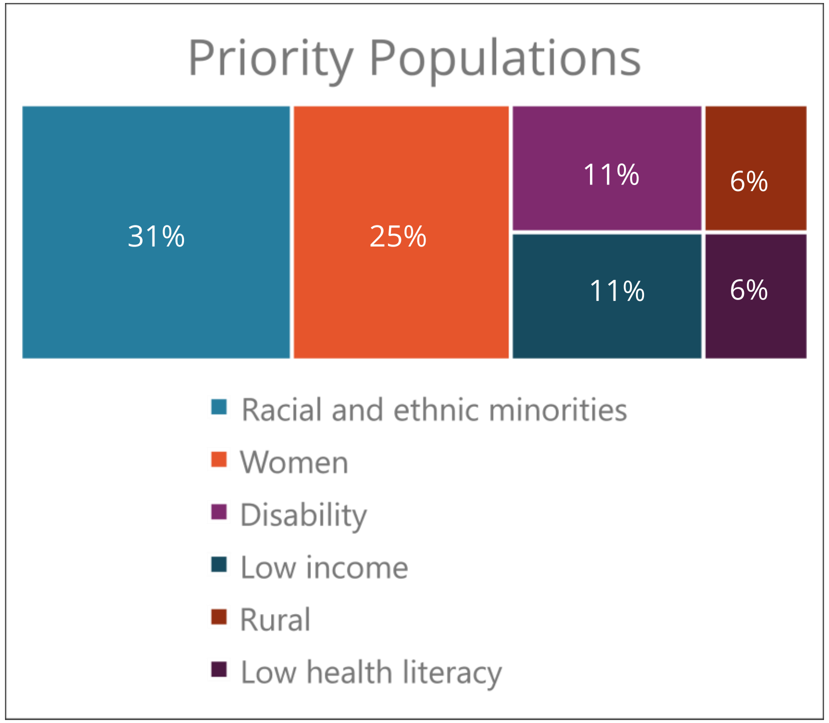


Others include:

- Kawasaki Disease
- Duarte Galactosemia
- Idiopathic Subglottic Stenosis
- Pediatric Transverse Myelitis
- Pediatric Crohn’s Disease
- Polyarticular Juvenile Idiopathic Arthritis
- Chiari Type I Malformation and Syringomyelia
- Systemic Scleroderma
- Eosinophic Esophagitis
- Myasthenia Gravis
- Lupus Nephritis
- Pulmonary Fibrosis
- Juvenile Spondyloarthritis
- Hydrocephalus
- Nontuberculous Mycobacterial (NTM) Lung Disease
- Non-CF Bronchiectasis
- Neuroendocrine Tumors
- Pediatric Chronic Kidney Disease
- Congenital Heart Defects

# Closer Look at the CER Awards





# Topics Studied

**Natural History** (n=2; $4.5M)

**Patient Self-Directed Care** (n=3; $8 M)

e.g., self-management strategies

**Shared Decision Making** (n=4; $5 M)

e.g., decision support tools & processes

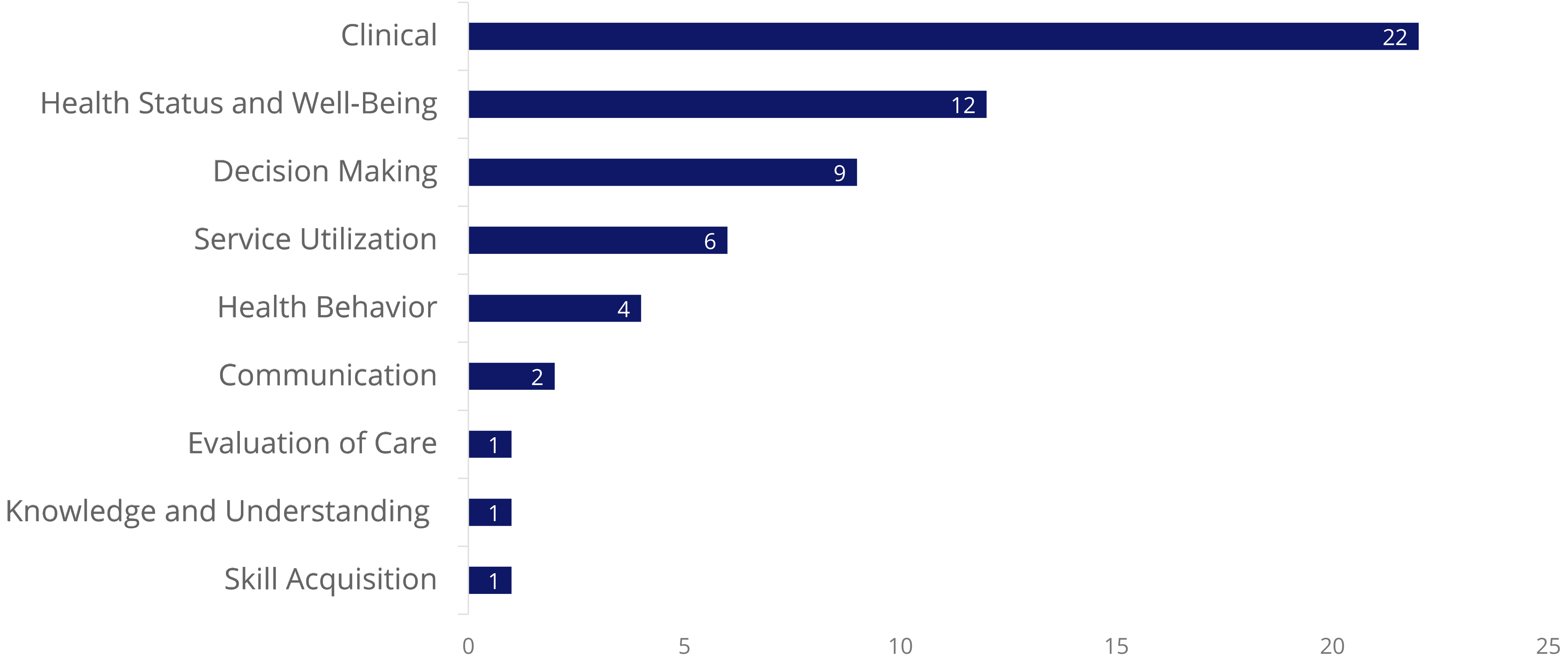
**Healthcare Service Delivery Models** (n=8; $3.5 M)

e.g., health service models

**Clinical Treatments** (n=17); $59 M

e.g., drug interventions

# Primary Outcomes by Theme



**Not mutually exclusive; As of November 2021*

# Cross-cutting CER Topics Relevant to Patients with Rare Diseases

## **9** studies focus on cross-cutting research topics

### Shared Decision Making

- Decision support tools
- Shared decision-making toolkit
- Personalized decision aids

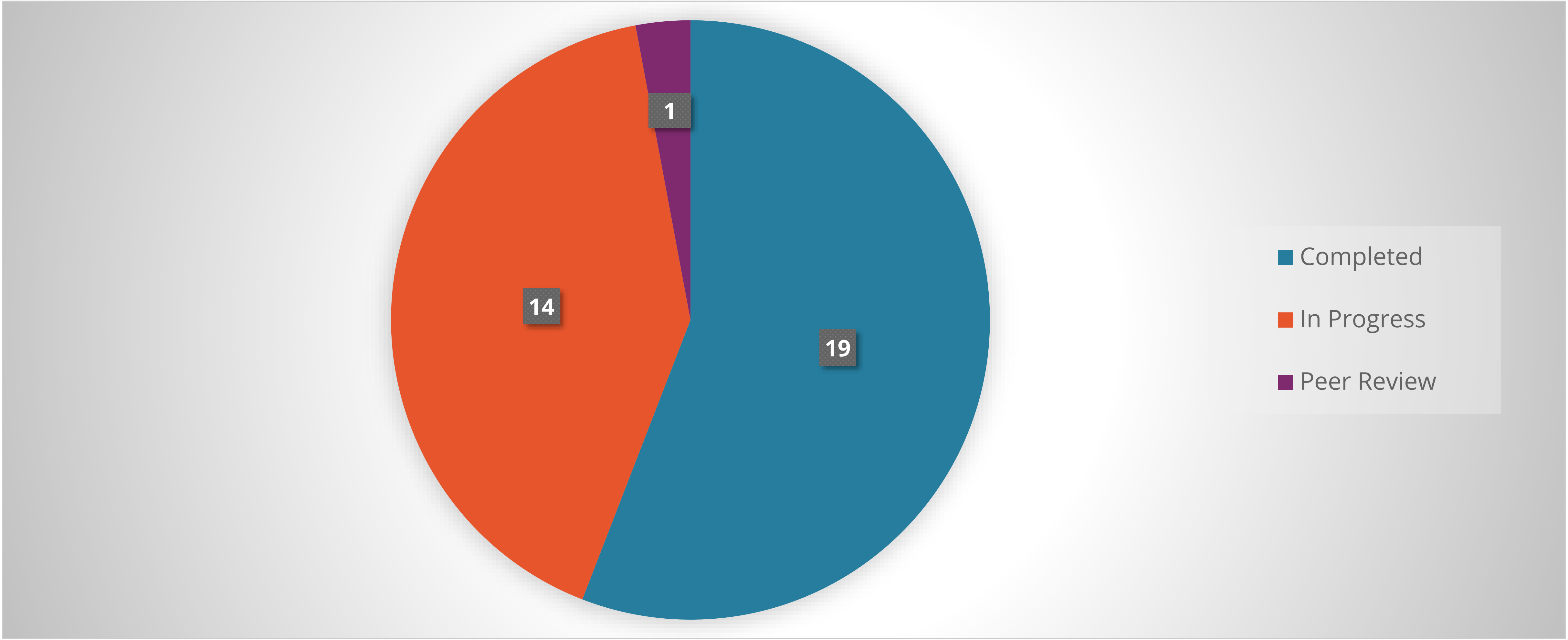
### Patient self-directed care

- Mobile interventions for pain management
- Online disease self-management tool

### Health service delivery models

- Interventions to improve treatment adherence
- Transitional care models
- Self-management support interventions

# Rare Disease CER Projects by Results Availability/Completion Status



STUDY PROFILE

# Tools and Information to Guide Choice of Therapies in Older and Medically Infirm Patients with AML

## Engagement

- Acute myeloid leukemia (AML) patients were given a comorbidity index to help them determine their best treatment options

## Potential Impact

- Could change practice by tailoring intensive treatments to those who can tolerate them rather than basing treatment on age, leading to a better quality of life during and after treatment

## Methods

- Observational study

Develops a comorbidity index that can predicts whether treatment will be successful based on characteristics other than age, the usual cutoff point for determining treatments, and gives physicians and families a better understanding of risks and benefits.

*Mohamed Lofty Sorror, MD, MS,*
*Fred Hutchinson Cancer Research Center*
*Seattle, WA*
*Assessment of Prevention, Diagnosis, and Treatment*
*Options, awarded May 2013*

Sorror ML, Storer BE, Fathi AT, Gerds AT, Medeiros BC, Shami P, Brunner AM, Sekeres MA, Mukherjee S, Peña E, Elsawy M, Wardyn S, Whitten J, Moore R, Becker PS, McCune JS, Appelbaum FR, Estey EH. Development and Validation of a Novel Acute Myeloid Leukemia-Composite Model to Estimate Risks of Mortality. JAMA Oncol. 2017 Sep 7. doi: 10.1001/jamaoncol.2017.2714. [Epub ahead of print] PubMed PMID: 28880971

STUDY PROFILE

# Comparative Effectiveness of CARRA Treatment Strategies for Polyarticular Juvenile Idiopathic Arthritis

## Engagement

- Two patient partners are members of the core research team. A stakeholder advisory committee, composed of patients, parents, pediatric rheumatology nurses, and research associates, will advise the research team on study design, engagement, and dissemination.

## Potential Impact

- Biologics are highly effective, but can have side effects and toxicity, so knowing when they should be started to produce the best outcomes is critical.

## Methods

- Prospective, observational cohort study

Juvenile idiopathic arthritis (JIA) is the most common pediatric rheumatic condition, affecting 1-4 in 1,000 children. The Start Time Optimization in PJIA (STOP-JIA), aims to improve the lives of polyarticular JIA patients by comparing the clinical effectiveness of three different strategies for the introduction of biologic therapy in achieving clinically inactive disease.

*Yukiko Kimura, MD*
*Hackensack University Medical Center*
*Hackensack, NJ*
*Assessment of Prevention, Diagnosis, and Treatment Options, awarded April 2015*

Kimura Y, Schanberg LE, Tomlinson GA, Riordan ME, Dennos AC, Del Gaizo V, Murphy KL, Weiss PF, Natter M MD, Feldman BM, Ringold S; CARRA STOP-JIA Investigators. The Childhood Arthritis & Rheumatology Research Alliance Start Time Optimization of Biologics in Polyarticular Juvenile Idiopathic Arthritis Study (STOP-JIA): A Comparative Effectiveness Study of CARRA Consensus Treatment Plans for Untreated Polyarticular JIA. Arthritis Rheumatol. 2021 Jun 8. doi: 10.1002/art.41888. Epub ahead of print. PMID: 34105312.

# Discussion Questions

- What are your reactions to the balance of the portfolio? What would an ideal balance look like to you?
- Are there other aspects of PCORI’s CER and Methods research portfolio that you would like further information on?
- What topics (excluding specific diseases) can be addressed by CER that are not being covered?

# BREAK (20 minutes)

We will return at 3:45 pm EST

PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

# Strategic Planning: Proposed Research Agenda

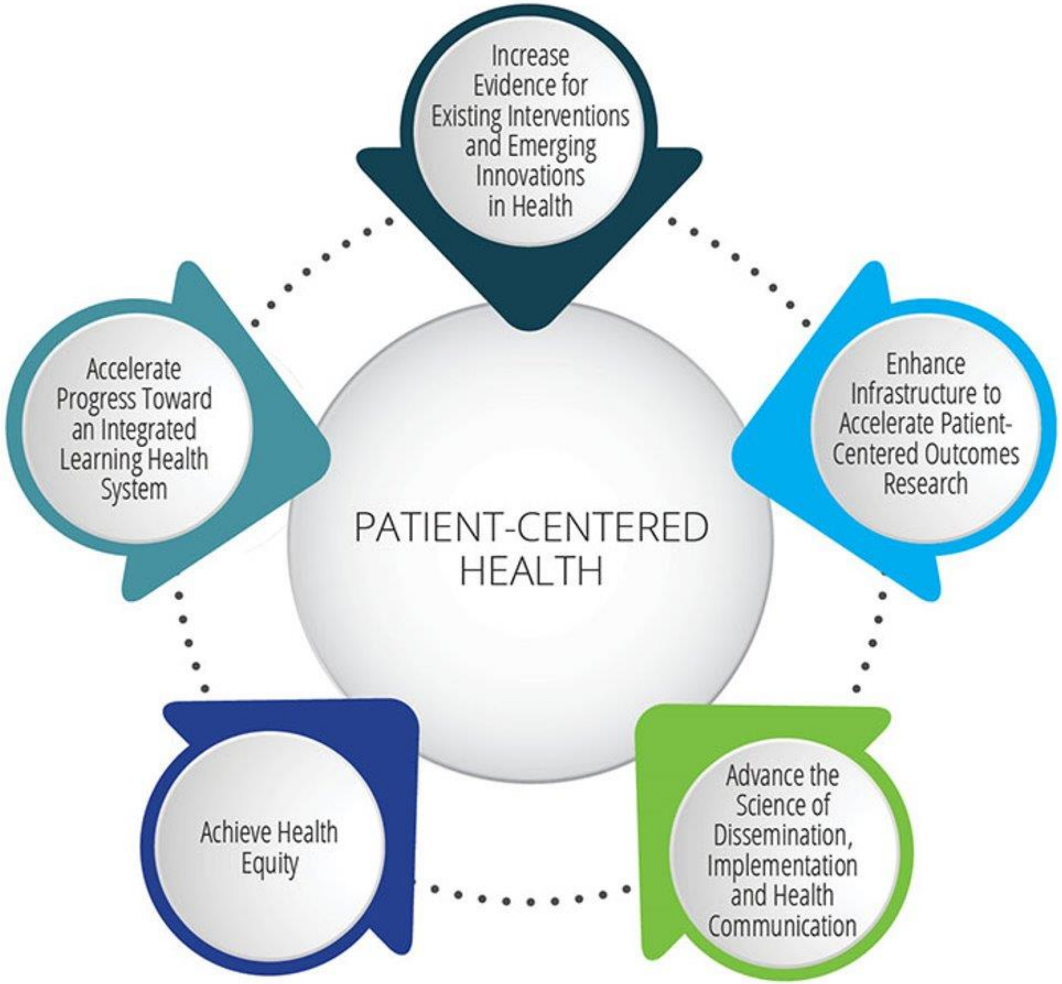
Marina Broitman, PhD

Associate Director, Peer Review

# Scope of Strategic Planning Activities

- **National Priorities for Health**
- **Research Agenda**
- Strategic principles for PCORnet® phase 3
- Methodology Committee focus for PCORI's next phase
- Commitment Planning and strategies to increase funding
- Scenario Planning based on the changes in landscape and environment
- Priorities from reauthorizing law
    - Maternal morbidity and mortality
    - Intellectual and developmental disabilities
    - Full range of outcomes data
- Monitoring progress and measuring success

# Adopted National Priorities for Health



PATIENT-CENTERED HEALTH

Increase Evidence for Existing Interventions and Emerging Innovations in Health

Enhance Infrastructure to Accelerate Patient-Centered Outcomes Research

Advance the Science of Dissemination, Implementation and Health Communication

Achieve Health Equity

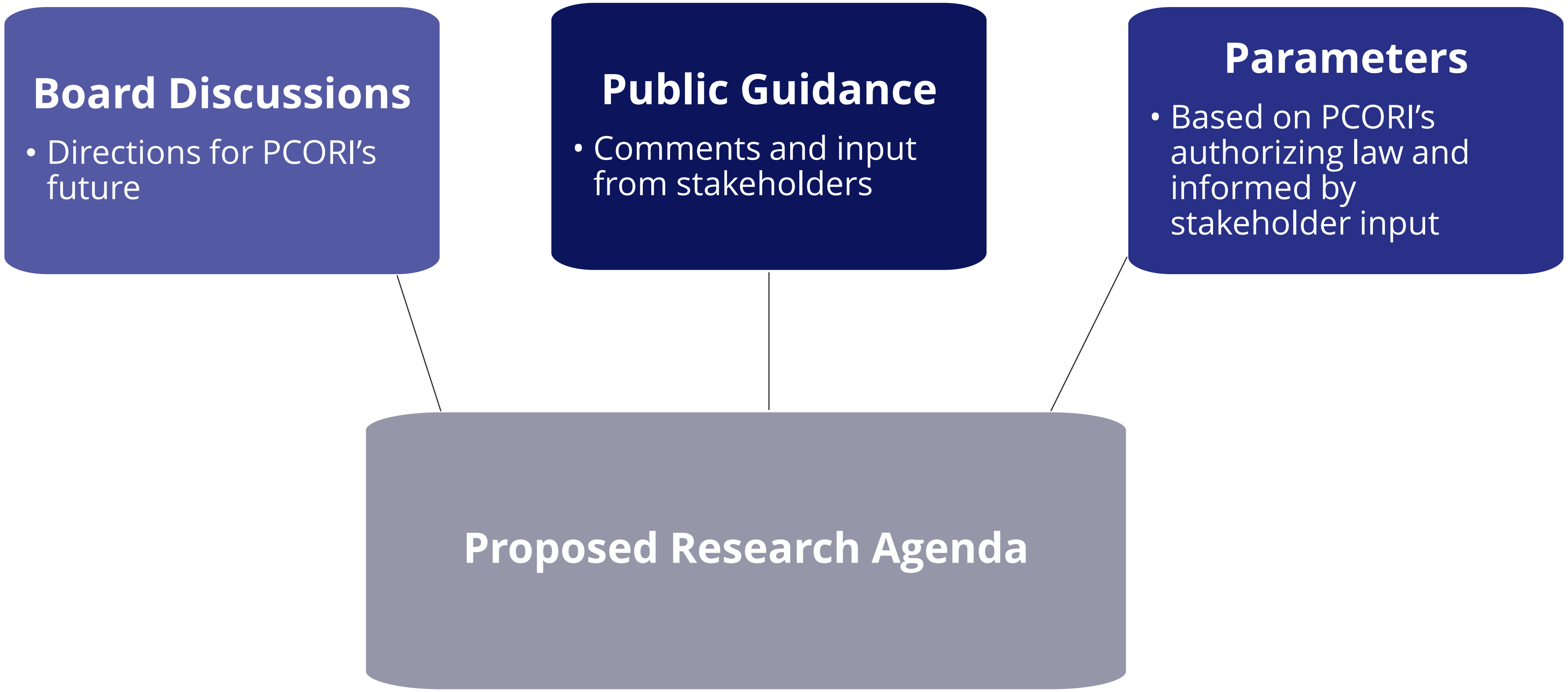
Accelerate Progress Toward an Integrated Learning Health System

# Research Agenda: A Component of the Strategic Plan

- The ***strategic plan*** centers on achieving the National Priorities for Health, and the Research Agenda is one major component of the plan. The strategic plan aims to describe all the various components and present a cohesive vision for PCORI's future activities.
- The ***Research Agenda*** provides a framework for achieving progress on the National Priorities for Health specifically through the strategy of funding comparative clinical effectiveness research (CER). The Research Agenda helps guide the development of continuously relevant ***Research Project Agenda*** via an on-going, stakeholder-engaged process.
- PCORI utilizes other strategies in addition to funding CER (for example, Dissemination & Implementation) to achieve the National Priorities for Health, and these will be described in other components of the strategic plan.

Intellectual and Developmental Disabilities (IDD)
Maternal Morbidity and Mortality (MMM)

# Creation of the Research Agenda



**Board Discussions**

- Directions for PCORI's future

**Public Guidance**

- Comments and input from stakeholders

**Parameters**

- Based on PCORI's authorizing law and informed by stakeholder input

**Proposed Research Agenda**

# Proposed Research Agenda

- Fund research that fills patient- and stakeholder-prioritized evidence gaps and is representative of diverse patient populations and settings
- Fund research that aims to achieve health equity and eliminate health and healthcare disparities
- Fund research that builds the evidence base for emerging interventions by leveraging the full range of data resources and partnerships
- Fund research that examines the diverse burdens and clinical and economic impacts important to patients and other stakeholders
- Fund research that focuses on health promotion and illness prevention by addressing health drivers that occur where people live, work, learn, and play
- Fund research that integrates implementation science and that advances approaches for communicating evidence so the public can access, understand, and act on research findings

# Proposed Research Agenda and Examples for Supporting Context

**Research Agenda Statement:**

Fund research that fills patient- and stakeholder-prioritized evidence gaps and is representative of diverse patient populations and settings

**Illustrative Examples**

- Research on topics and outcomes of high relevance and value to patient and stakeholder communities that generates timely evidence to inform decisions
- Research that recruits study participants that are representative of communities most affected by conditions being studied and are representative of the diversity of the nation in order to understand potential differences in treatment effects and outcomes across populations
- Research that involves investigators that reflect the population or community where the research is conducted and for whom the research is intended

**Research Agenda Statement:**

Fund research that aims to achieve health equity and eliminate health and healthcare disparities

**Illustrative Examples**

- Research across conditions, populations, and subpopulations on topics where disparities in health outcomes occur
- Research on innovations for health systems that drive practice change to promote equitable care and eliminate discriminatory health care practices
- Research that supports and develops the talent of diverse, early career investigators and the health care workforce

# Proposed Research Agenda and Examples for Supporting Context- Continued

## Research Agenda Statement:

Fund research that builds the evidence base for emerging interventions by leveraging the full range of data resources and partnerships

**Illustrative Examples**

- Research that leverages evidence synthesis and stakeholder engagement to meet the evidentiary needs of federal, state, and local policymakers and stakeholders
- Research on innovations identified through relationships with federal partners including the FDA, CMS, NIH, VA, CDC, and AHRQ
- Research using optimized methods of data capture that are inclusive of diverse sources of real-world information (including patient-provided data)

## Research Agenda Statement:

Fund research that examines the diverse burdens and clinical and economic impacts important to patients and other stakeholders

**Illustrative Examples**

- Research that engages patients, caregivers, and other key stakeholders in identifying important outcomes, unintended consequences, burden, and economic impacts
- Research that assesses the full range of factors that influence health outcomes, such as patient preferences, economic issues, practice variation, and disparities in the delivery of care

# Proposed Research Agenda and Examples for Supporting Context- Continued

## Research Agenda Statement:

Fund research that focuses on health promotion and illness prevention by addressing health drivers that occur where people live, work, learn, and play

**Illustrative Examples**

- Research in cross-sector collaboration with organizations with expertise in the social determinants of health (e.g., economic stability; education access and quality; health care access and quality; neighborhood and built environment; social and community context)
- Research on upstream topics such as social determinants of health, public health, and policies or programs that influence population-level health
- Research that examines the use of and investment in health-promoting resources in the community

## Research Agenda Statement:

Fund research that integrates implementation science and that advances approaches for communicating evidence so the public can access, understand, and act on research findings

**Illustrative Examples**

- Research that studies the effectiveness of ways to get information to the right people, at the right time, in the right way that addresses cultural and other tailoring, modes of communication, and appropriate messengers for specific populations
- Research on approaches for rapid dissemination of evidence to inform the immediate decisional needs of patients, clinicians, health systems, and other stakeholders
- Research on the effectiveness of implementation strategies that have potential for accelerating the uptake of evidence into practice
- Research that assesses methods for explaining uncertainty, applicability, and communicating risk, including approaches for tailoring these methods for diverse populations

# Hearing from the Panel

## Discussion Questions

1. How might the proposed Research Agenda meet future needs for PCORI's strategy of funding CER?
2. How could the proposed Research Agenda support a research portfolio that is inclusive of this panel's topics of interest?
3. Is there an important research area that you would like to see better reflected in the proposed Research Agenda?
4. What kinds of research portfolios will be important to support the specific Statements?

**Proposed Research Agenda**

- Fund research that fills patient- and stakeholder-prioritized evidence gaps and is representative of diverse patient populations and settings
- Fund research that aims to achieve health equity and eliminate health and healthcare disparities
- Fund research that builds the evidence base for emerging interventions by leveraging the full range of data resources and partnerships
- Fund research that examines the diverse burdens and clinical and economic impacts important to patients and other stakeholders
- Fund research that focuses on health promotion and illness prevention by addressing health drivers that occur where people live, work, learn, and play
- Fund research that integrates implementation science and that advances approaches for communicating evidence so the public can access, understand, and act on research findings

# Round Robin Brainstorming Session

# Acknowledgments and Recap

# Thank You!

# Adjourn