Advisory Panel on Rare Disease (RDAP) Virtual Meeting: Spring 2022

May 18, 2022

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

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 Spring 2022 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.

Meeting Agenda

Start Time	Agenda Items	Presenters & Discussion Facilitator
12:30 PM	Welcome, Introductions, and Setting the Stage	Mat Edick, Doug Lindsay
12:50 PM	Introduction of RDAP PCORI Staff & RDAP Program Staff Update	Nora McGhee, Fatou Ceesay
1:00 PM	Intellectual and Developmental Disabilities Update	Carly Khan, Doug Lindsay
1:30 PM	Uptake of Rare Disease Results	Heather Edwards, Mat Edick
2:00 PM	BREAK (15 minutes)	
2:15 PM	Building Capacity for Cross-Cutting Rare Disease Research	Mat Edick, Doug Lindsay
3:00 PM	Healthcare Cost and Value – Addressing the Full Range of Outcomes	Greg Martin, Kristen Giombi, Mat Edick
3:45 PM	BREAK (15 minutes)	
4:00 PM	Update on Strategic Planning: PCORI's Strategic Plan	Laura Lyman Rodriguez, Doug Lindsay
4:30 PM	Acknowledgments and Recap	Mat Edick, Doug Lindsay
4:45 PM	Farewell to Departing Members	Mat Edick
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

Engagement Program Updates

Fatou Ceesay, MPH

Program Officer, Engagement Awards

Meghan Berman, MPH

Program Associate II, Public and Patient Engagement

Eugene Washington PCORI Engagement Award Program

Building Capacity for Small Organizations to Engage in PCOR/CER

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Engagement Award Program Commitment

- Meet the needs of diverse stakeholders
- Expand our reach

Pre-Announcement Overview

The new Engagement Award: Building Capacity for Small Organizations to Engage in PCOR/CER funding opportunity will support two-year projects that enable small organizations and their communities to build capacity and skills to participate in the PCOR/CER process.

Pre-Announcement Overview

- The projects will leverage each awardee organization's unique perspectives and experiences to **build the knowledge, competencies, and abilities** of the organization and its community to be meaningful partners with researchers throughout all phases of the PCOR/CER process.

- Awardees will be required to participate in a **learning network** throughout the project period. The learning network will provide an opportunity for awardees to receive **technical assistance** on their projects, share expertise and experiences, and offer lessons learned.

- Two-year projects; up to $250,000 total (direct and indirect)

Eligibility
Applicant organizations:

- Must have an average yearly operating budget of $1.5 million or less over the past two fiscal years.
- Must be tax-exempt under section 501(c)(3) of the Internal Revenue Code *or* have a fiscal sponsor that is tax-exempt under section 501(c)(3) of the Internal Revenue Code.
- May not have received a total of over $100,000 through previous awards from the Eugene Washington PCORI Engagement Award Program. Funds received as a subcontractor on an award do not count towards this total.
- Must demonstrate a) current relationships with researchers and/or existing research networks; and b) current relationships with patients and other stakeholders who have a connection to, expertise in, or lived experience with, the topic area.

Release Date

Funding Announcement Release Date: **July 19, 2022**

Promotion of Funding Opportunity

- Goals:
  - Reach new audiences with connections to small organizations not yet familiar with Engagement Awards
  - Inform small organizations that were not successful with prior Engagement Awards applications
  - Inform small organizations that have received $100,000 or less in funding from prior Engagement Awards

Science of Engagement Initiative

Science of Engagement Initiative

Key Questions to Be Answered

- **What defines successful engagement, and for whom?**
- **How can engagement be measured? What indicates that a study is patient-centered?**
- **What are the approaches that support and achieve successful engagement, particularly for underrepresented populations?**
- **Under which conditions do approaches work best, and how should they be modified and resourced for different contexts, settings, and communities?**
- **Which approaches should be combined to achieve engagement aims?**

First PFA Priority Areas

Development and/or validation of measures, including rapid measure development, to capture structure/context, process, and outcomes related to both engagement and research, from both the stakeholder and investigator perspective.

Development and/or testing of engagement approaches to generate evidence on which specific aspects of engagement are most effective, either alone or in combination, and how effectiveness varies by context, particularly for underrepresented populations.

Notice of Upcoming PCORI Funding Announcement located here

Science of Engagement Initiative – Updated Timeline

Key Dates

ONLINE SYSTEM OPENS

- July 12, 2022; 9am (ET)

TOWN HALL

- July 19, 2022, 12 – 1pm (ET)

LETTER OF INTENT DEADLINE

- August 9 by 5pm (ET)

APPLICATION DEADLINE

- November 15 by 5pm (ET)

MERIT REVIEW

- Late February/early March 2023

AWARDS ANNOUNCED

- TBD

EARLIEST START DATE

- TBD

Funds and Project Period

FUNDS AVAILABLE: UP TO $4.5M

- Rapid Measurement Studies: Up to $1.5M
- Development and Testing of Engagement Approaches: Up to $3M

TOTAL DIRECT COSTS

- Rapid Measurement Studies: Up to $500,000
- Development and Testing of Engagement Approaches: Up to $1.5M

MAXIMUM PROJECT PERIOD

- Rapid Measurement Studies: Up to 2 years
- Development and Testing of Engagement Approaches: Up to 3 years

Registration for Science of Engagement PFA Applicant Town hall here

General Program Updates

Nora McGhee, PhD

Senior Program Officer, Clinical Effectiveness and Decision Science

General Program Updates

- **Save the Date**: The 2022 PCORI Annual Meeting will be held Wednesday, October 26 – Thursday, October 28, 2022. Registration for the meeting will open in the summer.
  - The meeting will include both in-person and virtual attendance as PCORI continues to closely monitor the situation with COVID-19 (coronavirus) and variants, with your safety being a top priority.
  - PCORI is pleased to offer financial scholarships for PCORI Ambassadors and patient and caregiver stakeholders to participate in-person at the 2022 PCORI Annual Meeting. Learn more and apply.
- **The next RDAP meeting will be held on October 25, 2022,** the day before the Annual Meeting. Panel members are expected to have the option to either attend in person or attend the meeting virtually.
- Advisory panel applications are under review – we anticipate new members will join us for the Fall 2022 Meeting.

Follow up from Winter 2021 Meeting

- Research Agenda modified to be more inclusive of historically excluded populations such as rare disease communities.

- Brainstorming sessions last meeting highlighted the huge diagnostic struggles related to rare diseases and suggested we explore solutions. Our literature review suggests large number of research studies ongoing in this area as well as active new tool development.

Intellectual and Developmental Disabilities (IDD) - Update

Carly Khan, PhD, MPH, RN

Associate Director, Healthcare Delivery and Disparities Research

Origins of IDD Prioritization

2019 – Congress reauthorizes PCORI – Amending legislation to specify two new national priority areas:

- Maternal mortality
- Individuals with intellectual and developmental disabilities

Implementation of mandate will include:

- A **long-term commitment** to priority areas of investment
- Ongoing opportunities for engagement to identify stakeholder driven topics and patient-centered outcomes

This means IDD is set as a national priority area by law, thus a long-term priority area of investment through broad funding announcements, special areas of emphasis funding announcements and topic specific funding announcements

Long Term Investment
IDD as a Research Priority

- PCORI has a dedicated IDD Workgroup that was created to guide PCORI's efforts in meeting the legislative mandate of IDD as a research priority.
- Over the ensuing years, this Workgroup will partner with stakeholders to develop and implement the research agenda to provide evidence to help neurodiverse individuals get the best care for them.
- To contact our IDD workgroup, please email us at IDDPCORI@pcori.org

Prior Discussion of IDD Priority with RDAP Members

- The RDAP members were last briefed on this new national priority during the Winter 2020 meeting
- RDAP members discussed the following relevant topical areas that cut across IDD and rare disease future research needs:
  - Sleep
  - Transitions from pediatric to adult care
  - Importance of addressing social determinants of health
- RDAP members also recommended different rare disease organizations that PCORI could connect with

PCORI’s Approach and Work To Date in Supporting IDD Research

A Multipronged Approach to Improving Health and Quality of Life for Neurodiverse Individuals

Funding high-priority research

- Full range of research awards and evidence synthesis products
- Identification of short and long-term evidence gaps and priorities

Building capacity for PCOR

- Engagement awards
- Community-building projects
- Workforce awards
- PCORnet infrastructure awards

Dissemination and implementation activities

- Dissemination and implementation of PCORI-funded projects
- Other D&I activities

Enhancing efficiency and research design

- Understanding and providing guidance on methodologic issues
- Increasing engagement in research

Intellectual and Developmental Disabilities:

Portfolio Investments to Date

SO FAR, PCORI HAS AWARDED

$80 MILLION TO FUND **64**

comparative clinical effectiveness research studies and research support projects with a focus on **intellectual and developmental disabilities**

AS OF December 2021

*PCORI has also funded six Methods and Infrastructure awards related to IDD

Funding High Priority Research:

Focused Funding to Address the Needs of Individuals with IDD

- Special Areas of Emphasis (SAEs):
  - *Past*: Improving Care for Individuals with Intellectual and/or Developmental Disabilities Growing into Adulthood
  - *Present (22C2)*: Caregiver-Delivered Interventions for Intellectual and Developmental Disabilities
- Targeted PCORI Funding Announcement (tPFA): Comparative Effectiveness of Interventions Targeting Mental Health Conditions in Individuals with IDD
- IDD identified as a research priority in several of PCORI's standard funding announcements
- Additional research topics are currently under development

Background of Currently Open Cycle 1 2022 tPFA:

IDD and Co-Occurring Mental Health

- **Priority Research Question:** What is the comparative effectiveness of evidence-based approaches (e.g., specific pharmacologic and behavioral interventions) that address mental health in individuals with IDD?
  - Pharmacological, behavioral, other nonpharmacological or combination interventions administered via appropriate delivery modalities

Total Direct Costs:
Up to $3M

Maximum Project Period:
3 years

- PCORI has allocated a total of up to **$40 million** for this Targeted PFA

Building Capacity for PCOR/CER in IDD:

Engagement Award Special Cycle

Special Cycle Funding Announcement to support projects that enable organizations and communities to build their capacity and skills to participate across all phases of the PCOR/CER process on topics that address health outcomes related to IDD on topics such as:

- Addressing health equity and advancing IDD health outcomes for vulnerable populations
- Leveraging community-based and patient-centered models of care delivery
- Challenges related to clinical care delivery and access
- Telehealth solutions
- Transition to adulthood
- Caregiver needs and access to wraparound support
- Patient needs and preferences

51 projects totaling over $10M to date

Stakeholder Engagement

Ongoing Stakeholder Engagement

- To date, we have engaged 99 stakeholders representing:
  - Patients and self-advocates
  - Caregivers
  - Clinicians
  - Researchers
  - PCORI awardees
- Represented within these stakeholders are groups working in autism, Down syndrome, ADHD, and other IDD

Discussion

Discussion

- Are there other topical areas of focus relevant to both IDD and rare disease?
- Are there methodological considerations relevant to rare disease research that may be relevant to IDD research?
- Are there stakeholder groups PCORI should consider connecting with?

Contact the IDD Workgroup

IDDPCORI@pcori.org

Uptake of Rare Disease Study Results

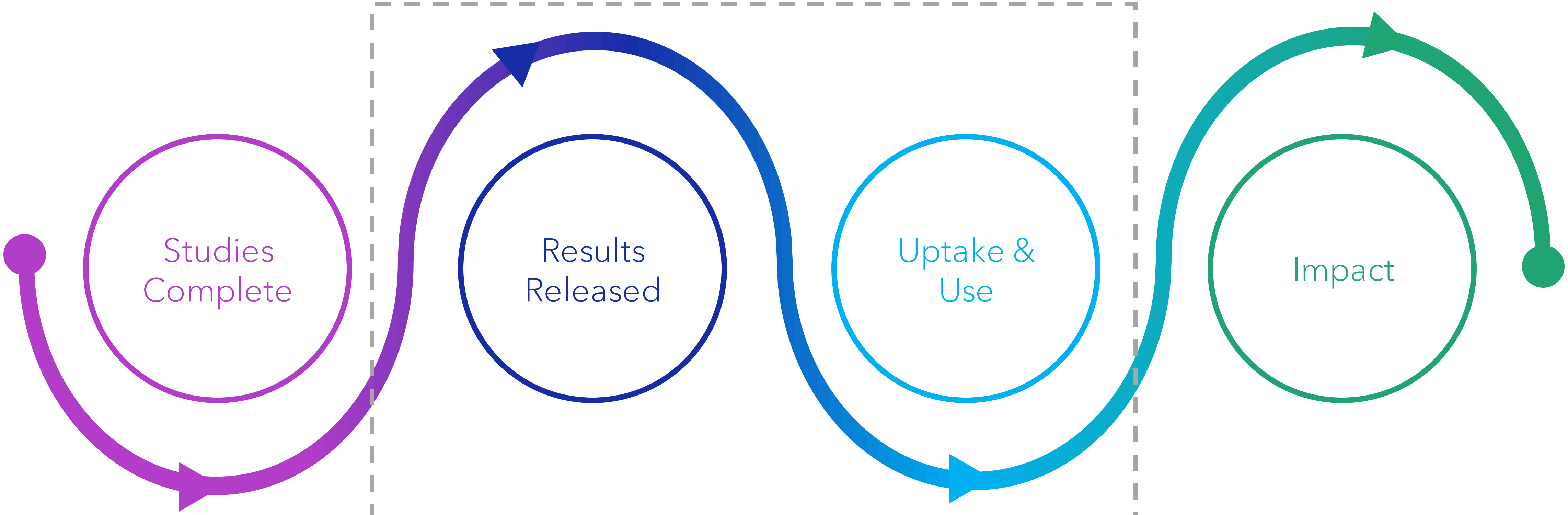
Heather Edwards

Associate Director, Executive Office, Evaluation and Analysis

Agenda

- Report on Results Release, Use and Uptake
- Seek insights about patient and patient groups’ use of research findings

Roadmap to Impact



Studies Complete

Results Released

Uptake & Use

Impact

Completed studies have posted abstracts on pcori.org

EXAMPLES

Publications; "Altmetrics"; Results returned to participants

EXAMPLES

Patient/consumer education materials; Adoption into new settings

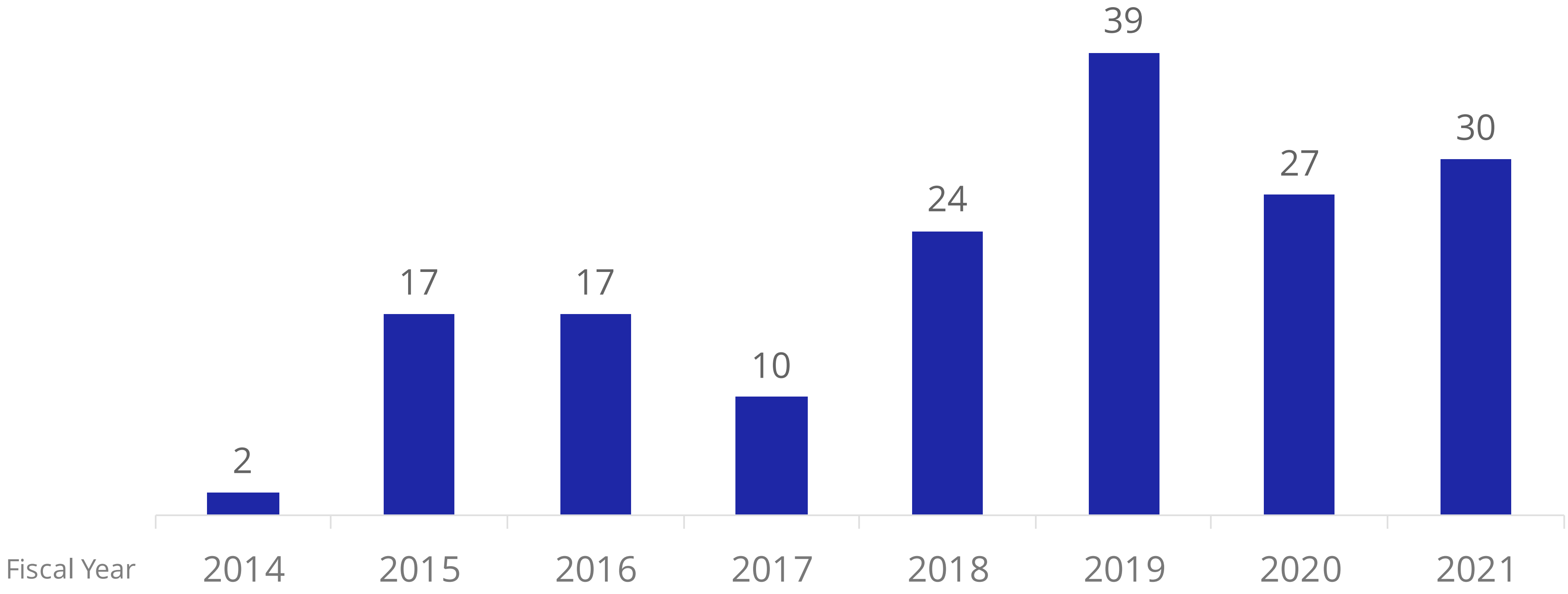
Improved health:
-- Care
-- Decisions
-- Outcomes

A more detailed description of this path to impact can be found at https://www.pcori.org/sites/default/files/PCORI-Evaluation-Analysis-Dissemination-to-Use-to-Impact-1600x1200.jpg

Rare Disease (RD) Portfolio Overview

Type of Project	Awarded	Completed
Comparative Effectiveness Research	37	21
PCOR Methods Research	4	3
Engagement Awards	71	52

PCORI RD Projects Have Published 169 Articles in Total



Analysis includes all peer reviewed articles associated with PCORI research and support awards through 12/31/2021.
Note that there is often a lag between publishing & identification, so we continue to find publications from prior years.

Results Released through Publications

Comparative Effectiveness Research studies about RD have

- 128 total publications
- 17 publications of CER results

THE LANCET
Child & Adolescent Health

Infliximab versus second intravenous immunoglobulin for treatment of resistant Kawasaki disease in the USA (KIDCARE): a randomised, multicentre comparative effectiveness trial

Jane C Burns, MD • Samantha C Roberts, MPH • Adriana H Tremoulet, MD • Feng He, MS • Beth F Printz, MD
Negar Ashouri, MD • et al. Show all authors

CER Results Articles Have Been Published on a Variety of Conditions

- Sickle Cell Disease
- Resistant Kawasaki Disease
- Polyarticular Juvenile Idiopathic Arthritis
- Duarte Galactosemia
- Idiopathic Subglottic Stenosis
- Lupus
- Scleroderma
- Spinal Cord Injury and Spina Bifida
- Pediatric Crohn’s Disease
- Hydrocephalus

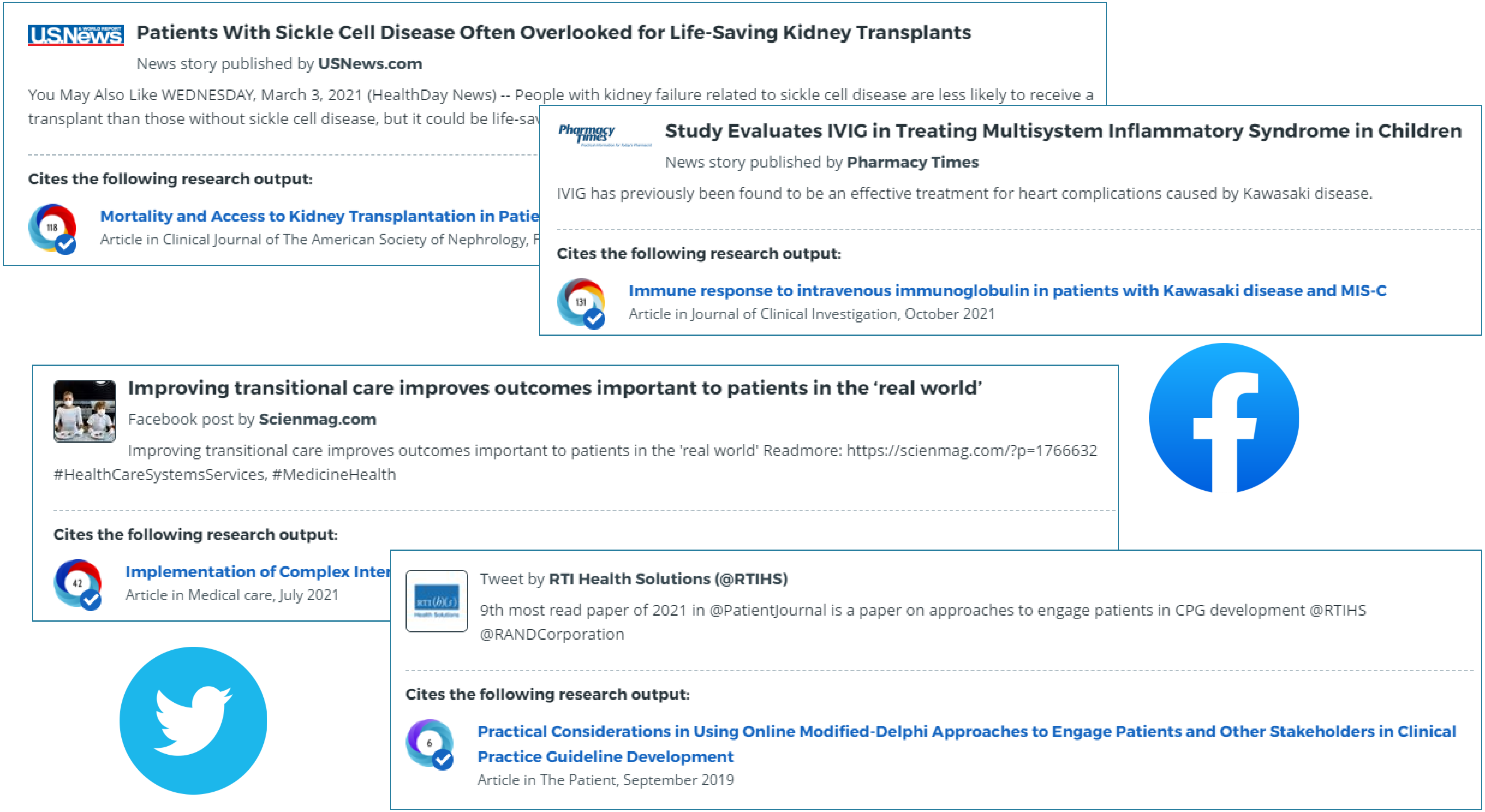
Some studies published multiple articles with primary findings. Also, PCORI has funded multiple studies on some topics.

79% of Rare Disease Projects' Findings Have Been Shared Through Public-Facing Channels

Of the 169 articles associated with this portfolio, 134 were mentioned

- 1,890 times on Twitter
- 76 times on Facebook
- 120 times in news stories

Data thru Q1FY2022
(12/31/2022)



Patients With Sickle Cell Disease Often Overlooked for Life-Saving Kidney Transplants

News story published by **USNews.com**

You May Also Like WEDNESDAY, March 3, 2021 (HealthDay News) -- People with kidney failure related to sickle cell disease are less likely to receive a transplant than those without sickle cell disease, but it could be life-sav

Cites the following research output:

Mortality and Access to Kidney Transplantation in Patie

Article in Clinical Journal of The American Society of Nephrology, F

Study Evaluates IVIG in Treating Multisystem Inflammatory Syndrome in Children

News story published by **Pharmacy Times**

IVIG has previously been found to be an effective treatment for heart complications caused by Kawasaki disease.

Cites the following research output:

Immune response to intravenous immunoglobulin in patients with Kawasaki disease and MIS-C

Article in Journal of Clinical Investigation, October 2021

Improving transitional care improves outcomes important to patients in the 'real world'

Facebook post by **Scienmag.com**

Improving transitional care improves outcomes important to patients in the 'real world' Readmore: https://scienmag.com/?p=1766632 #HealthCareSystemsServices, #MedicineHealth

Cites the following research output:

Implementation of Complex Inter

Article in Medical care, July 2021

Tweet by **RTI Health Solutions (@RTIHS)**

9th most read paper of 2021 in @PatientJournal is a paper on approaches to engage patients in CPG development @RTIHS @RANDCorporation

Cites the following research output:

Practical Considerations in Using Online Modified-Delphi Approaches to Engage Patients and Other Stakeholders in Clinical Practice Guideline Development

Article in The Patient, September 2019

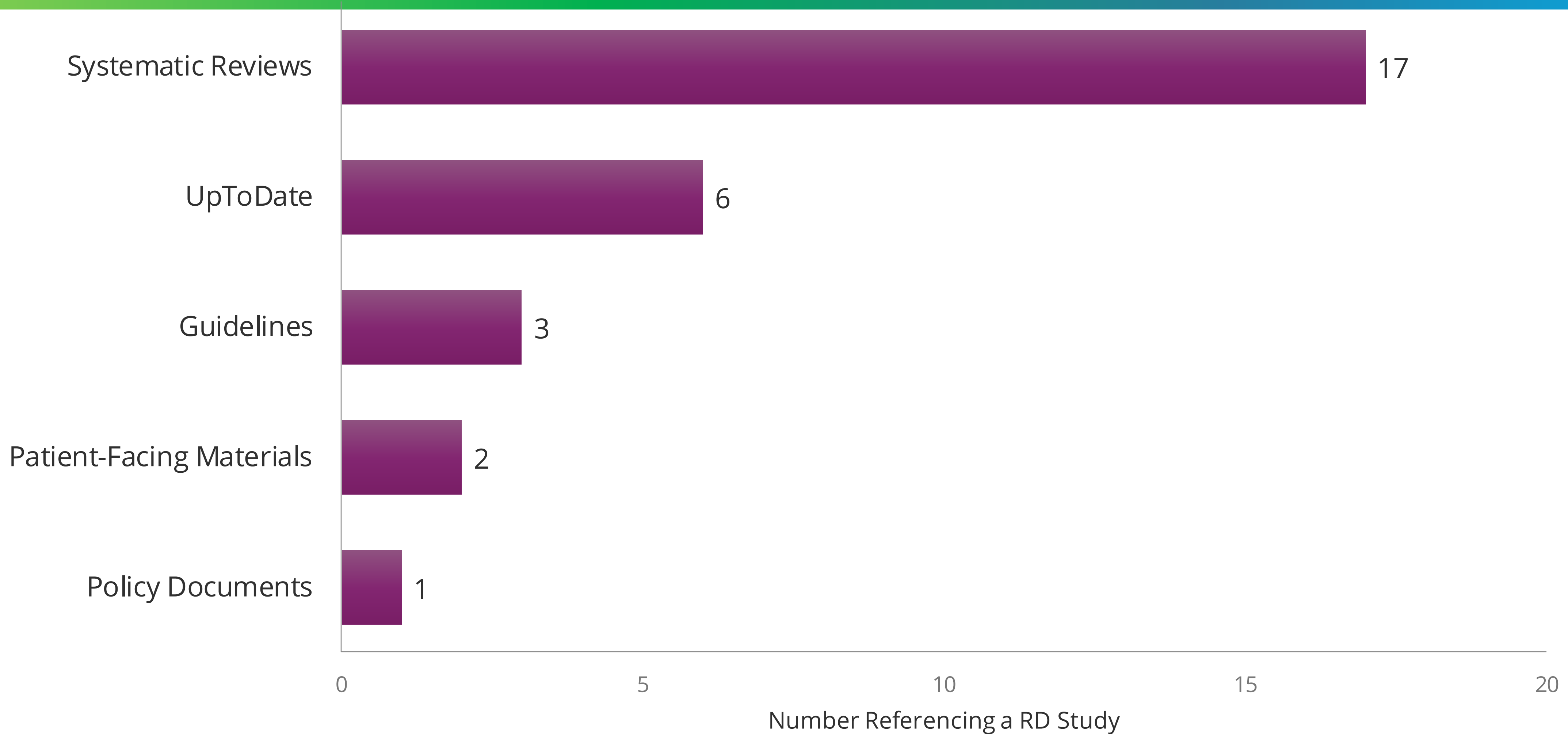
Uptake and Use of Results from PCORI-Funded Studies:

Current Metrics

- Incorporation into
  - Systematic reviews
  - Patient/consumer education materials
  - Graduate Medical Education or Continuing Medical Education
  - Practice guidelines
  - Decision making infrastructure (e.g., decision aids, clinical reference tools)
  - Payer policies
  - Institutional, local, state, and national policy

- Adoption of study findings
  - Into applied settings
  - Outside the study setting

Uptake and Use of Results from PCORI-Funded Studies on Rare Disease



Questions

- What are your reactions to these findings about the Rare Disease portfolio?

- What else would you like to learn about Results Release, Uptake, and Use in the future?

Opportunities to Better Understand and Detect Uptake and Use

How can we expand our approach to better understand how patients and patient groups may be using results?

- How do patients and patient groups access and share information about their conditions?
- How might PCORI detect and systematically track these uses?

- Incorporation into
  - Systematic reviews
  - Patient/consumer education materials
  - Graduate Medical Education (GME) or Continuing Medical Education (CME)
  - Practice guidelines
  - Decision making infrastructure (e.g., decision aids, clinical reference tools)
  - Payer policies
  - Institutional, local, state, and national policy

- Adoption of study findings
  - Into applied settings
  - Outside the study setting

BREAK (15 minutes)

We will return at 2:15 pm EST

PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

https://mphi.org/our-teams/center-for-strategic-health-partnerships/

in Center for Strategic Health Partnerships

f Center for Strategic Health Partnerships

@CSHPatWork

Building Capacity for Cross-Cutting Rare Disease Research

MATHEW J EDICK, PHD

MJEDICK@MPHI.ORG

Disclosures

I have no conflicts of interest

From 2013-2016, my center was the Midwest program office for PCORI's Pipeline to Proposal funding mechanism

I am a member of PCORI's Rare Disease Advisory Panel

I do not work for, or speak on behalf of the Patient Centered Outcomes Research Institute (PCORI)

All views and opinions expressed are my own

Funding for this work

- Health Resources and Services Administration (HRSA) of the U.S. Department of Health and Human Services (HHS) under grant number UH7MC30775, Midwest Genetics Network for $1,800,000 (0% financed with nongovernmental sources).

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- Newborn Screening Translational Research Network (“NBSTRN”), which is funded by the Eunice Kennedy Shriver National Institute of Child Health and Human Development, National Institutes of Health Award #HHSN275201300011C

- Health Resource and Services Administration (HRSA) Maternal and Child Health Bureau (MCHB) Regional Genetic and Newborn Screening Services Collaboratives, Heritable Disorders Program through grants to: Region 2 - New York Mid-Atlantic Consortium for Genetic and Newborn Screening Services (NYMAC) (H46MC24094), Region 4 Midwest Genetics and Newborn Screening Collaborative (H46MC24092), Region 5 Heartland Genetic Services Collaborative (H46MC24089), and Region 6 - Mountain States Genetics Regional Collaborative (H46MC24095).

- Patient-Centered Outcomes Research Institute (PCORI) Eugene Washington PCORI Engagement Award #3835-MPHI

- Patient-Centered Outcomes Research Institute (PCORI) Eugene Washington PCORI Engagement Award #6611-MPHI

- Patient-Centered Outcomes Research Institute (PCORI) Eugene Washington PCORI Engagement Award #EACC-18475

- Patient-Centered Outcomes Research Institute (PCORI) Intermediate Funders to Provide Operational, Programmatic, and Fiscal Agent Services to Pipeline to Proposal Awardees Contract (PCO-ENGAWD2014)

Meet the Team

CSHP operates at the interface of researchers, clinicians, public health professionals, payers, patients, families, community members, funders, and any other stakeholder groups to identify barriers and develop strategies to overcome them


MAT


JANE


AMY


ANGIE


DANIELLE


DRISCILLA


KAITLIN


KATHRYN


KELSEY


KRISTEN


NANETTE


NICOLE


SHANNAH

MPHI®

Center for Strategic Health Partnerships

Engaging Stakeholders | Increasing Access to Care | Improving Health Outcomes

Everyone Has a Story to Tell…

And Peoples' Stories Shape Who They Are and What's Important to Them.

Meet my son, Elijah (December 2006)

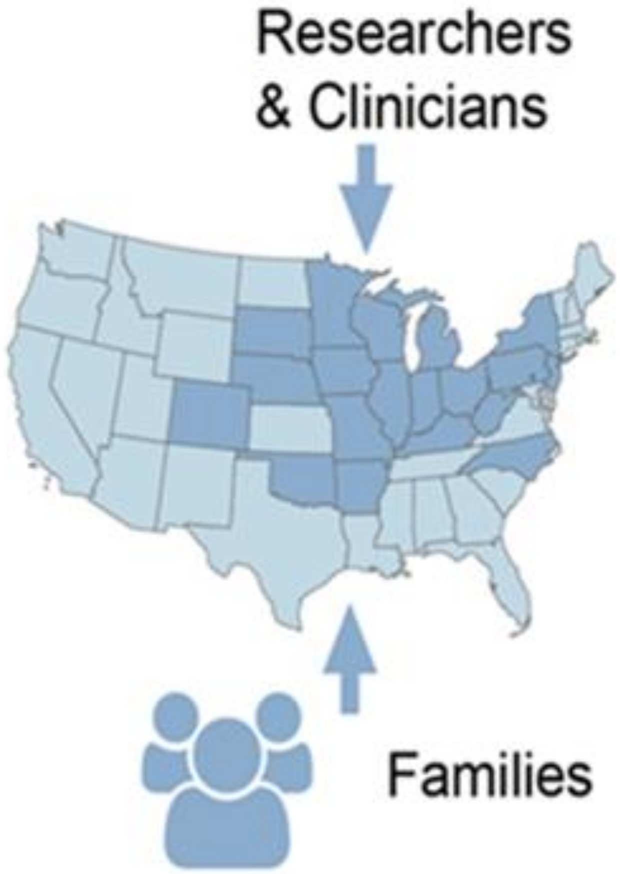
A New Perspective On Research

"Never again will I do research that is not important to the stakeholders most affected by the answer."

What Exactly Does it Mean to Engage Stakeholders?

Three Projects, Many Lessons Learned

Inborn Errors of Metabolism Collaborative (IBEMC)

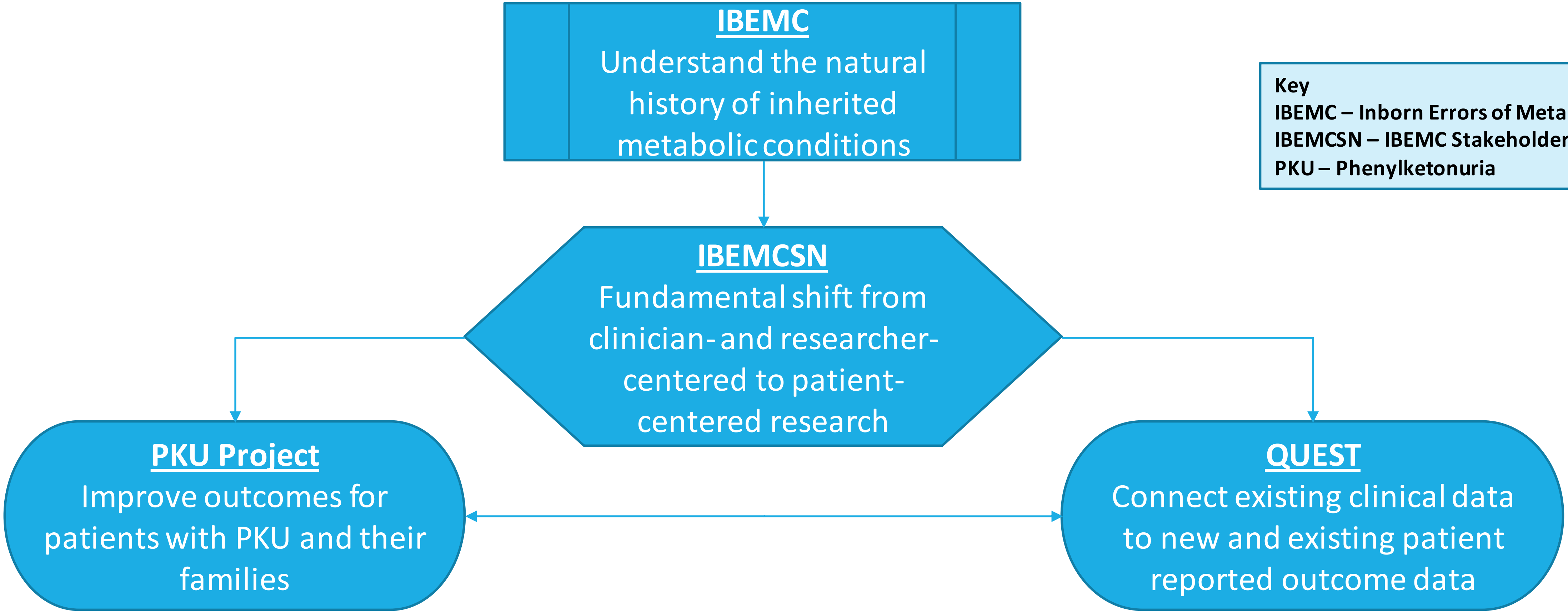


Natural History Study (2007-present)

- **25** Clinics Enrolling Patients
- **2154** Patients Enrolled
- **7234** Clinical Visits Recorded
- **77** Rare Disorders Represented

Berry SA, et al. Genet Med. 2016 Dec;18(12):1276-1281.

IBEMC Stakeholder Network Projects



IBEMC
Understand the natural history of inherited metabolic conditions

IBEMCSN
Fundamental shift from clinician- and researcher-centered to patient-centered research

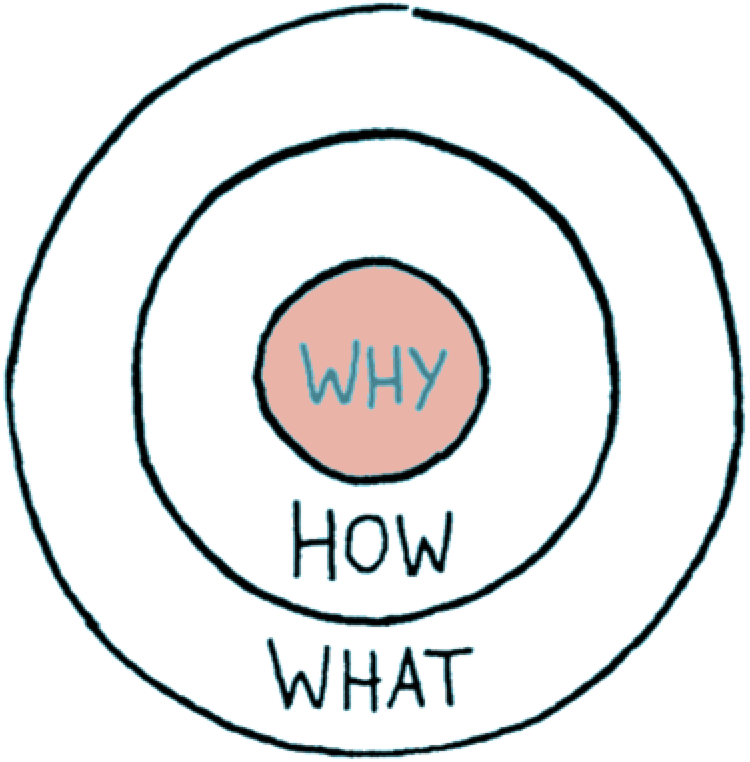
PKU Project
Improve outcomes for patients with PKU and their families

QUEST
Connect existing clinical data to new and existing patient reported outcome data

Key
IBEMC – Inborn Errors of Metabolism
IBEMCSN – IBEMC Stakeholder Network
PKU – Phenylketonuria

IBEMC Stakeholder Network

We recognize that patients and their families have **experience** and **expertise** critical for identifying the most important research questions, and for determining how to answer them based on **their** specific needs



“We Should not be guessing what patients need, we should be asking them”

Victory Circle

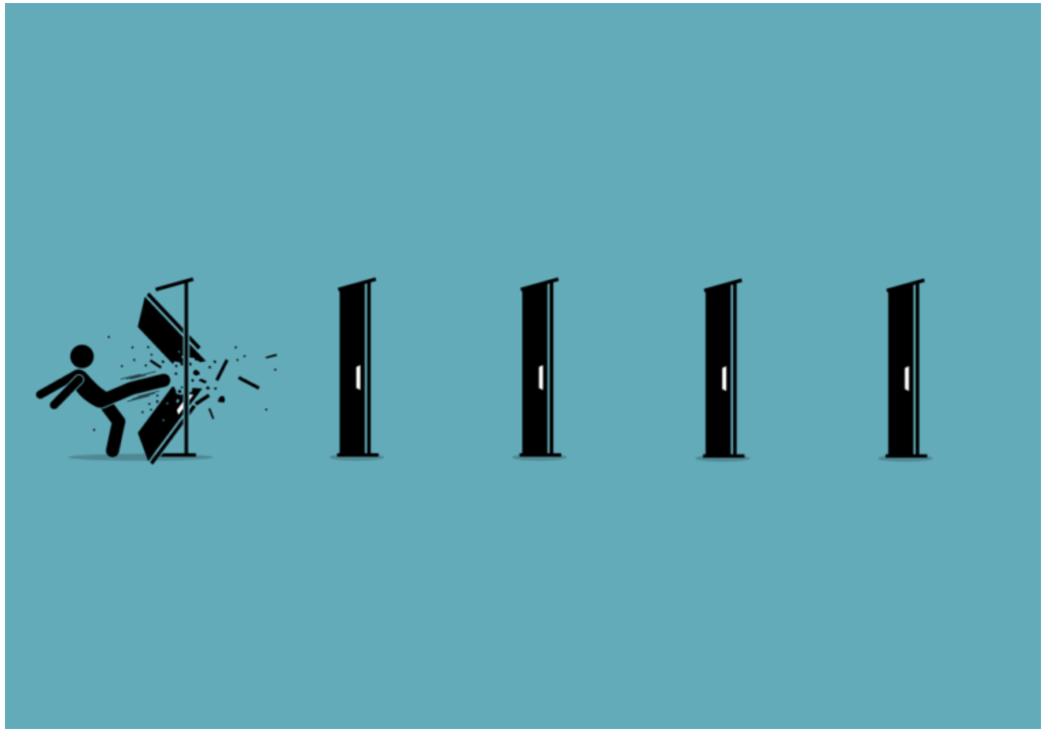
fund-able
* research question(s)
* outcome measures
Refine an idea for research
* partners identified
* funding source(s) identified
* Amt of funding

What does Success look like?

commitment

We will develop a quality research proposal(s) driven by patients + families, to inform treatment decisions for PKU

Notable Barriers Highlighted by Reviewers



Evidence of need was insufficient

Evidence of efficacy of the interventions was insufficient

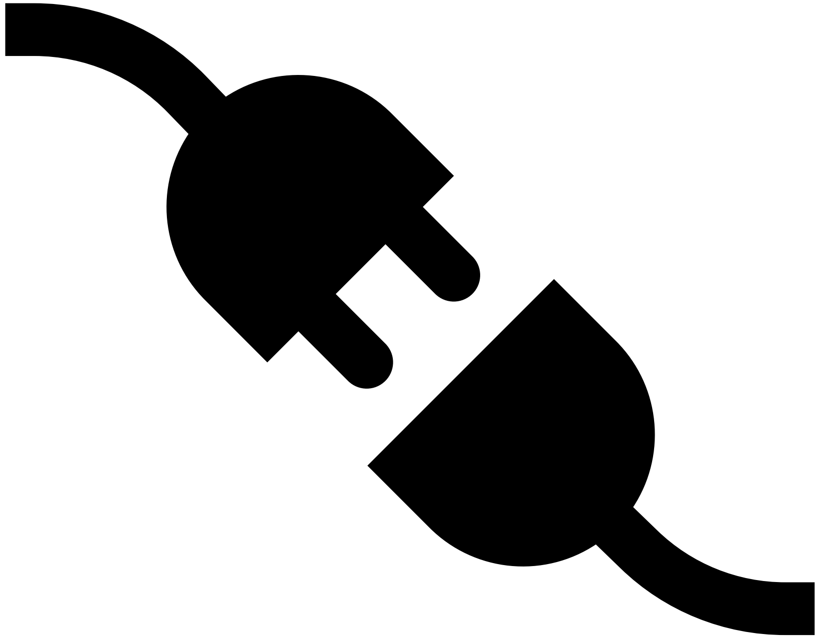
No buy-in from payers on this project

Insufficient sample size

THESE CHALLENGES ARE COMMON ACROSS MANY RARE DISEASES

Conception of the RaRE Project

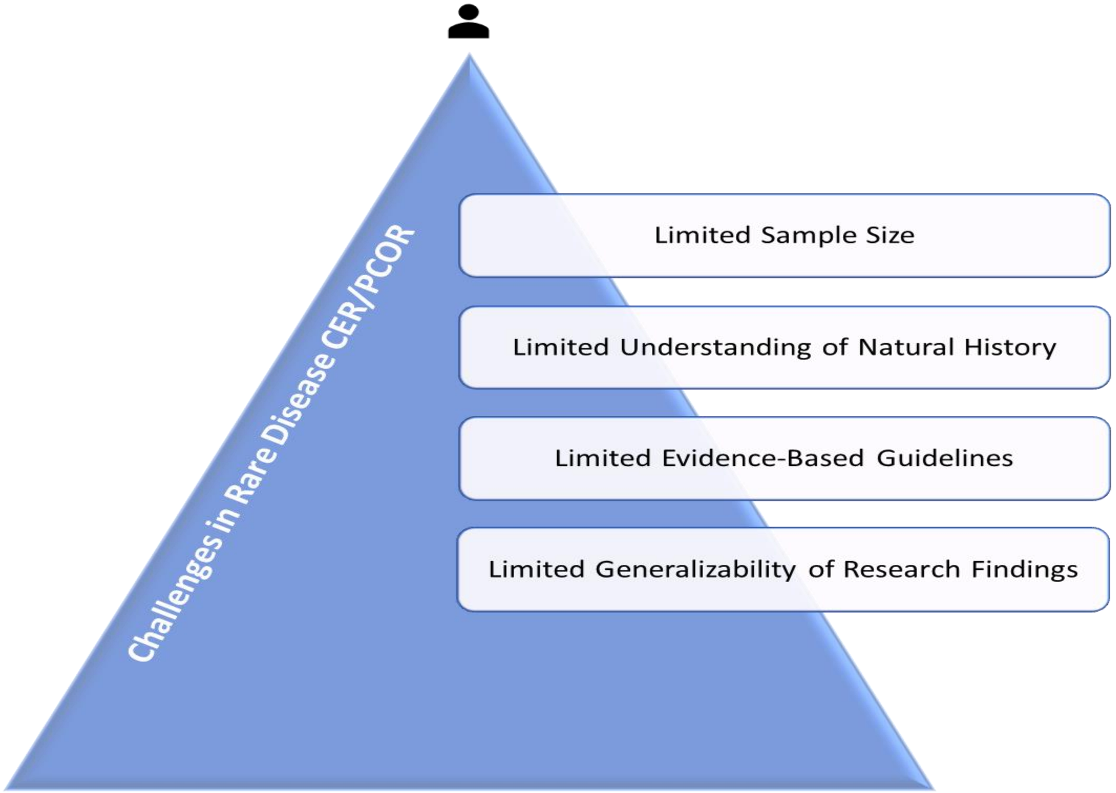
I interrupt this presentation to bring you a shameless plug for joining the PCORI Ambassador community



https://www.pcori.org/engagement/engage-us/pcori-ambassador-program

Barriers to Conducting Rare Disease Comparative Effective Studies

Rare Disease Research is Challenging



Limited Sample Size

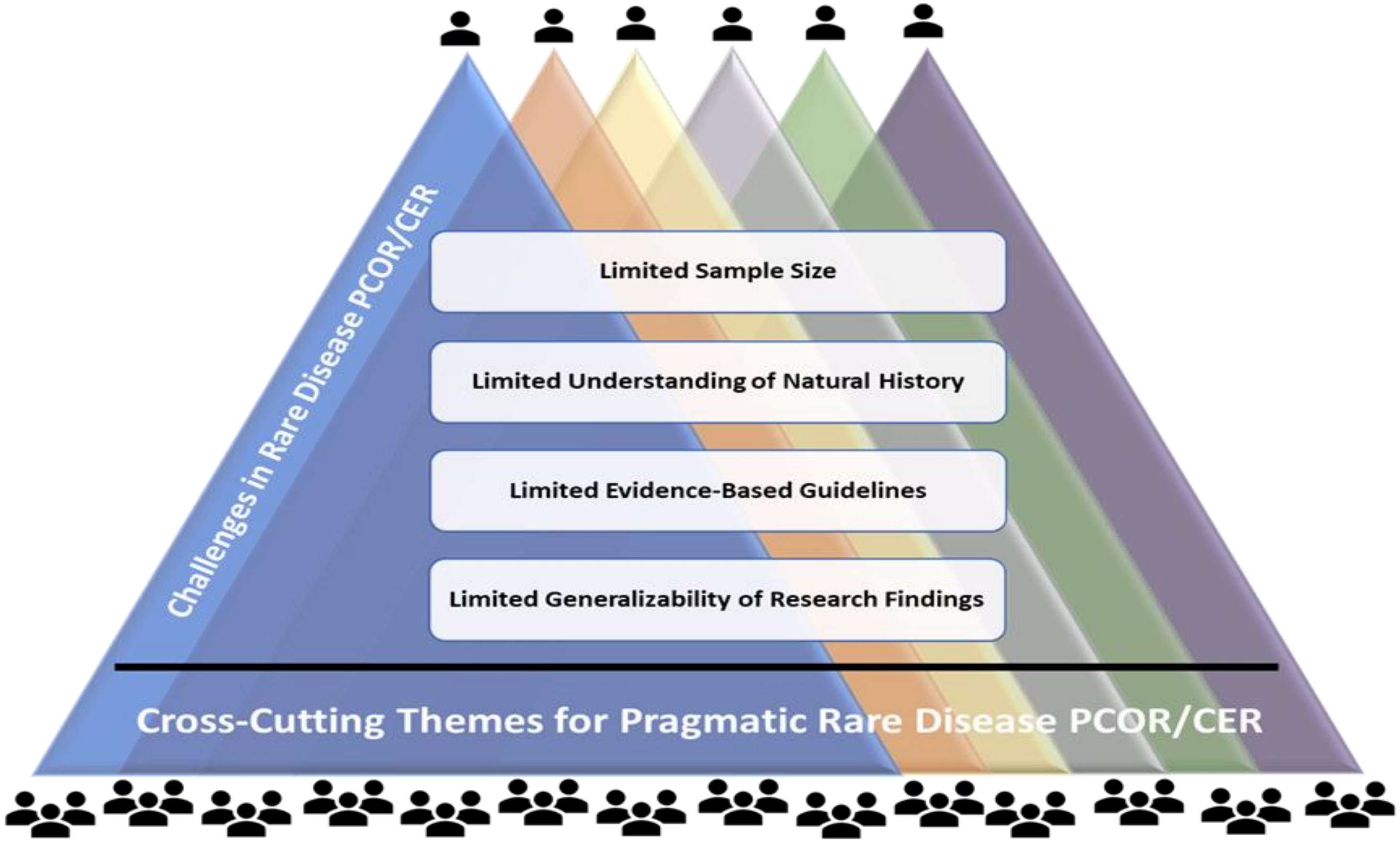
Limited Understanding of Natural History

Limited Evidence-Based Guidelines

Limited Generalizability of Research Findings

Typical Model:
Focused on MANY aspects of a SINGLE condition.

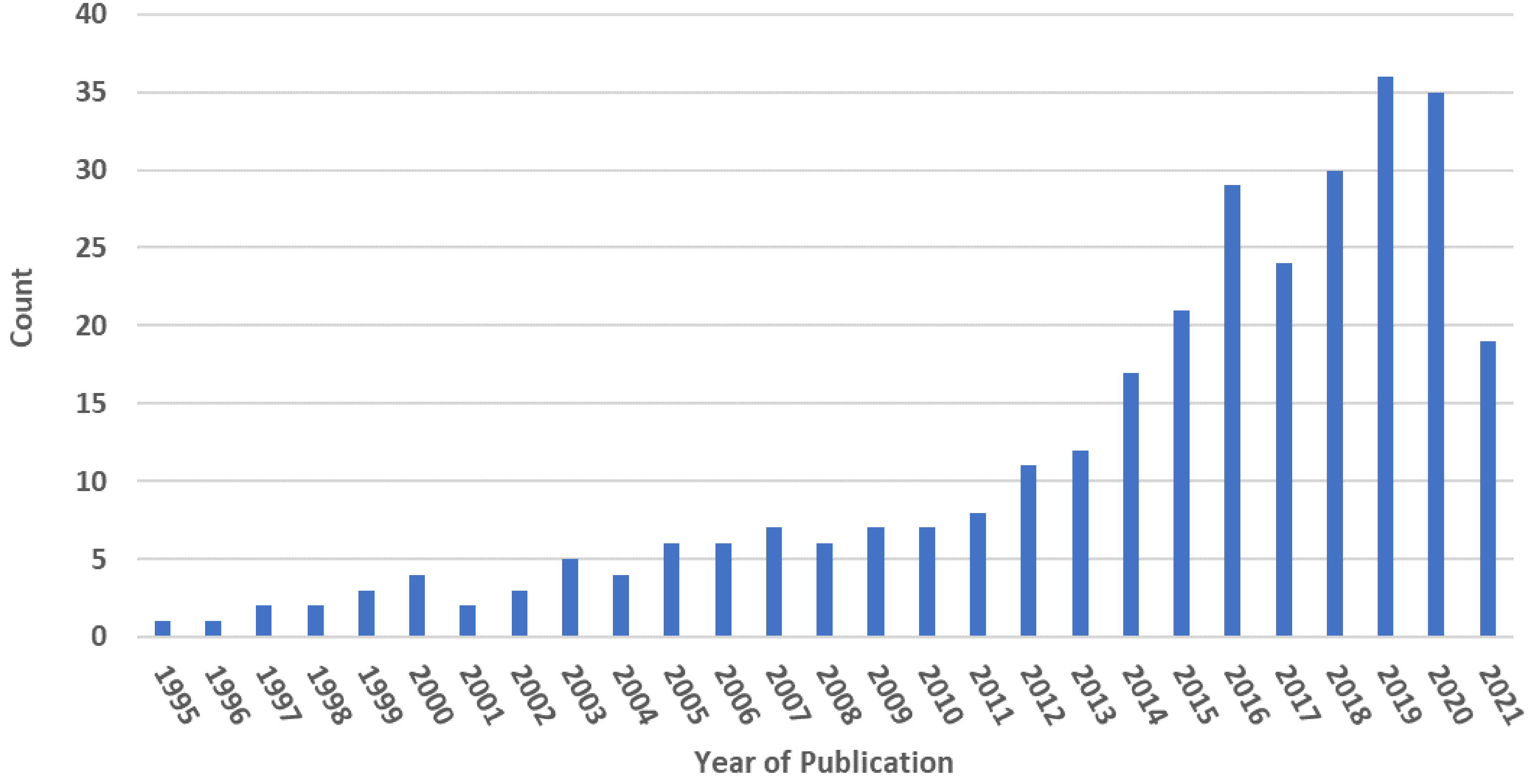
Strategy to Overcome the Barriers and Compete for PCORI Research Funding



Typical Model:
Focused on MANY aspects of a SINGLE condition.

Cross-cutting Model:
Focused on a SINGLE aspect of MANY rare diseases.

Journal articles published with rare disease and mental health as a key words



What about cross-cutting research studies that investigate and impact multiple rare diseases in a single study?

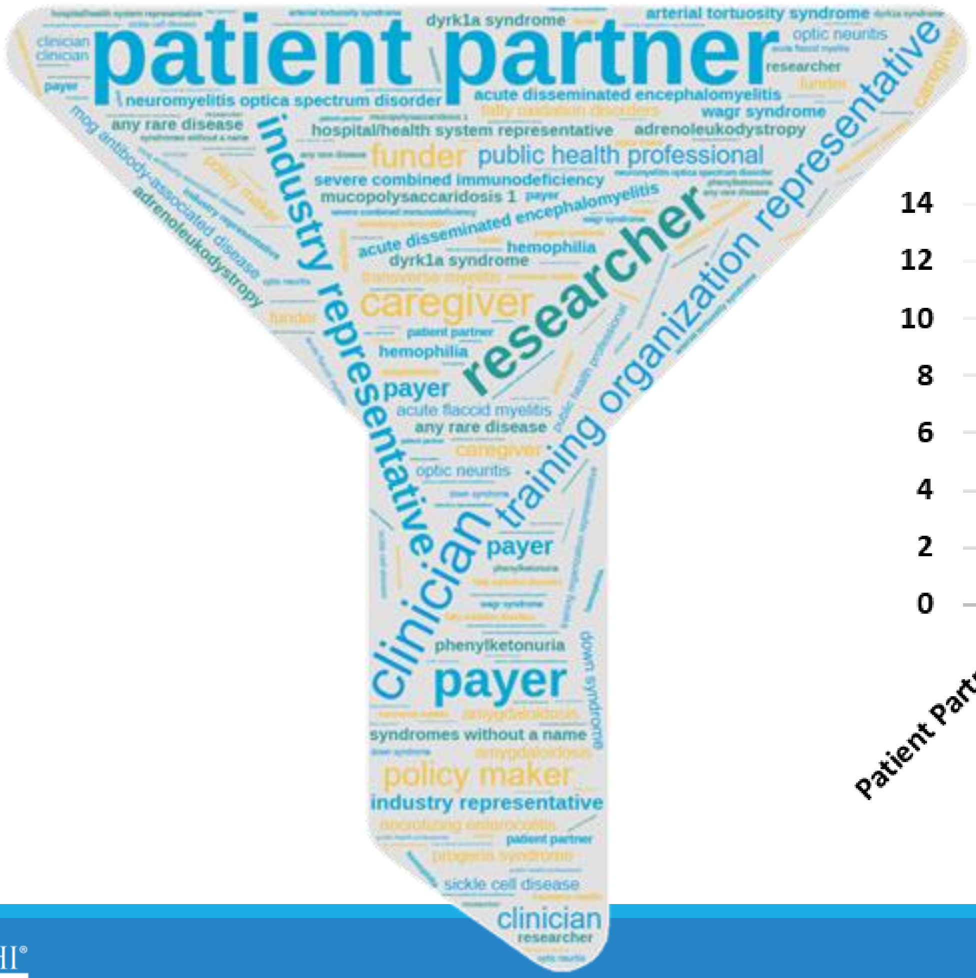
Rare Disease and **R**esearch **E**ngagement (RaRE)

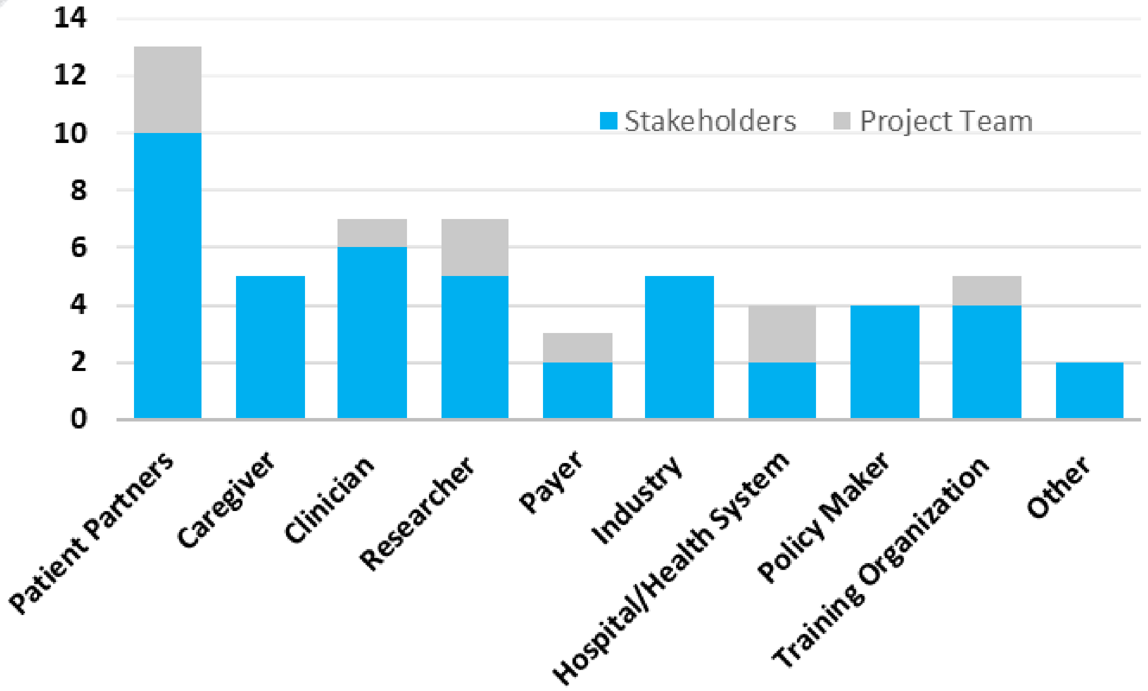
- Build a national network of diverse rare disease stakeholders interested in advancing patient centered outcomes research

- Identify cross-cutting patient-centered outcomes and research questions for comparative effectiveness research studies

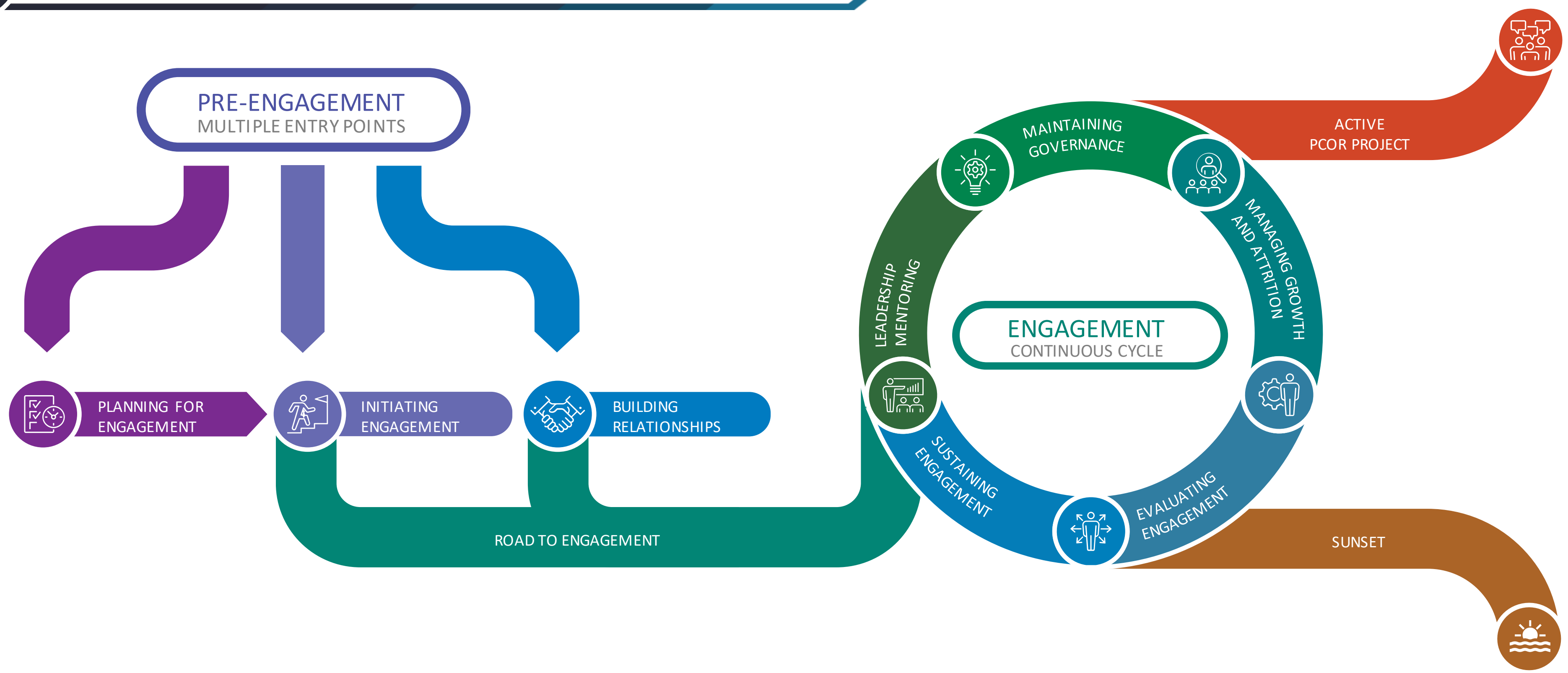
- Develop a comprehensive engagement model, or roadmap, to enhance collaborations across rare disease communities to implement and sustain robust stakeholder engagement

A Diverse Set of Rare Disease Stakeholders Participated





Roadmap for Rare Disease Stakeholder Engagement



PRE-ENGAGEMENT
MULTIPLE ENTRY POINTS

PLANNING FOR ENGAGEMENT

INITIATING ENGAGEMENT

BUILDING RELATIONSHIPS

ROAD TO ENGAGEMENT

ENGAGEMENT
CONTINUOUS CYCLE

MAINTAINING GOVERNANCE

MANAGING GROWTH AND ATTRITION

EVALUATING ENGAGEMENT

SUSTAINING ENGAGEMENT

LEADERSHIP MENTORING

ACTIVE PCOR PROJECT

SUNSET

GOALS FOR PRE-ENGAGEMENT

PLANNING FOR ENGAGEMENT

- Understand why engagement is important
- Establish team buy-in
- Address bias and other engagement barriers
- Identify common threads that unite rare disease stakeholders

INITIATING ENGAGEMENT

- Approach with curiosity and acknowledge limitations
- Identify gatekeepers and community connectors

BUILDING RELATIONSHIPS

- Create a shared vision
- Prioritize transparency, honesty, trust, and co-learning
- Acknowledge stakeholder expertise
- Break down silos

GOALS FOR ENGAGEMENT

LEADERSHIP MENTORING

- Develop new leaders from the rare disease community
- Model zero-tolerance for tokenism
- Prioritize resources for training and leadership development

MAINTAINING GOVERNANCE

- Establish collaborative problem solving and team-driven decision making
- Adopt equitable policies to guide operations
- Embrace transparency

MANAGING GROWTH AND ATTRITION

- Create a safe and supportive team environment
- Develop authentic relationships among team members
- Recognize the emotional aspects of the rare disease journey

EVALUATING ENGAGEMENT

- Identify opportunities for continuous quality improvement
- Facilitate data-driven decision making to guide engagement efforts

SUSTAINING ENGAGEMENT

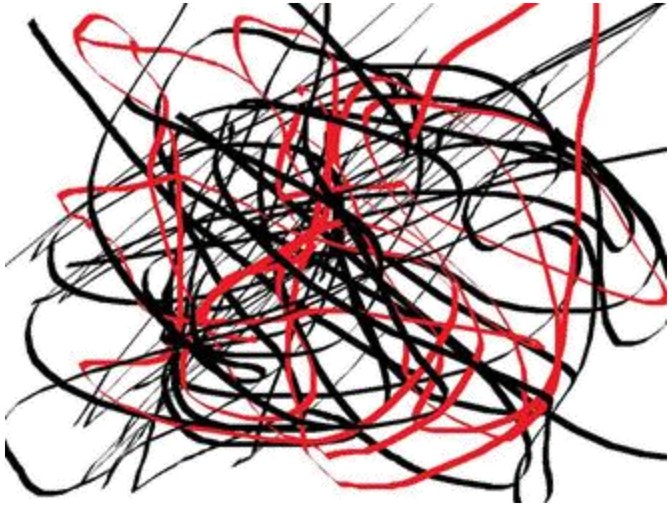
- Reduce time to mobilize diverse rare disease stakeholders
- Maintain authentic relationships with stakeholders to increase the impact of rare disease PCOR

Goals of Evaluating Engagement

Identify opportunities for continuous quality improvement

Facilitate data-driven decision making to guide engagement efforts

“Engagement is messy” – Jane Pilditch, RaRE Co-Lead and Patient Partner



Barriers:

- PCORI Methodology Standards related to evaluating engagement are limited
- Robust evaluation of engagement within the context of engagement awards will likely be cost prohibitive
- Tools to measure engagement from other disciplines (CBPR, Education Research, etc may be effective but there is limited demonstration of applying these tools in in PCOR

Goals of Sustaining Engagement

Reduce time to mobilize diverse rare disease stakeholders

Maintain authentic relationships with stakeholders to increase the impact of rare disease PCOR

“Authentic engagement is built on trust. Without trust, engagement quickly becomes disengagement.” – Mat Edick, RaRE Project Lead

Barriers:

- Inconsistent expectations imposed by funders is damaging to trust/relationships
- Funding specifically for engagement is limited, especially in the absences of an active research project
- Lack of a firm foundation

Cross-Cutting Rare Disease PCOR - Is the Juice worth the Squeeze?

Facilitators	Barriers
Synergy of shared resources.	Feasibility due to financial and temporal constraints.
Encourages diverse partnerships	Organizational challenges.
Expanded Funding opportunities	Limited funding
Generalizable Results	Condition-specific nuances may be lost or overlooked

Key Collaborators

- Patients and Families
- Cindy Cameron, PhD
- Janice Bach, GC
- Sue Berry, MD
- Nancy Leslie, MD
- Loren Pena, MD, PhD
- Jerry Feldman, MD
- Sandra VanCalcar, RD
- Sally Hiner
- Shaohui Zhou, PhD
- Liz Noe
- Kerie Hughes
- Marijata Daniel Echols, PhD
- Gina Gembel
- Julia Moore, PhD
- Kristen Reese
- Jennifer Canvasser
- Matt Cheung, PharmD
- Uday Deshmukh, MD
- Alissa Jorgenson
- Christina Love
- Ting Pun
- Zohreh Talebizadeh, PhD
- >75 additional IBEMC Partners
- >40 additional Rare Disease Stakeholders


STEP UP for DOWN SYNDROME
SPARTANS WILL.
MASON EQUESTRIAN TEAM
DIRT BIKE
ATHLETICS

Healthcare Cost and Value

Addressing the Full Range of Outcomes

Greg Martin
Acting Chief Engagement and Dissemination Officer, Office of the Chief Engagement and Dissemination Officer (CEDO)

Kristen Giombi, PhD
Economist, Health Economics,
RTI International

Agenda

Agenda

- Overview of PCORI Activity
  - Greg Martin, Acting Chief Engagement and Dissemination Officer
- Current Learnings
  - Olga Khavjou, RTI
  - Kristen Giombi, RTI
- Discussion

Overview

Framework for Activities

PCORI Approach to Healthcare Cost and Value: Framework for Activities

Collection of the Full Range of Outcomes

PCORI can ensure the appropriate and relevant data are identified and collected in PCORI awards

- Support broadening of PCORI guidance to awardees through literature reviews, patient & stakeholder focus groups to clarify types of potential burdens, costs, and economic impacts to be included in PCORI-funded research, and data sources
- Develop guidance for capture of specific types of potential burdens or costs consistent with a patient-centered perspective, as a basis for PCORI guidance to awardees and the field

Informing the Value Conversation

PCORI can help advance patient-centered approaches to the value conversation by supporting understanding of stakeholder perspectives on what constitutes "patient-centered value"

- Conduct a landscape review of current stakeholder statements, perspectives, and frameworks around value
- Convene patient and stakeholder groups to identify the components or attributes necessary in "patient-centered value"

Supporting Policy Priorities

PCORI can consider undertaking specific activities that contribute to priority policy goals (i.e., generating evidence to inform healthcare value discussions)

- Consider placing an emphasis on research focused on emerging technologies/therapies
- Consider building upon evidence synthesis capabilities to provide more timely information

Update on Activities and Deliverables

Informing the Value Conversation

Landscape Review

- How are different stakeholder communities defining “patient-centered value”?
- Where are the similarities and where are the differences?

Value Attributes

Category	Attribute	Patients/ caregivers	Clinicians	Policymakers	Payers	Purchasers	Life Sciences Industry	Hospital/ Health System	Researchers/ Funders	Number of Stakeholders
Life and social impact	Emotional/mental status/well being	🟩							🟩	**2**
	Fatigue	🟩							🟩	**2**
	Physical abilities/well being/independence	🟩							🟩	**2**
	Insurance value (physical and financial risk)								🟩	**1**
	Future planning	🟩						🟩		**2**
	Fear of rejection by family/society	🟩								**1**
	Support network	🟩						🟩		**2**
	Relationship with family/peers	🟩								**1**
	Participation in social activities	🟩								**1**
	Social history (cultural barriers and religious beliefs)	🟩						🟩		**2**
		36	**22**	**7**	**10**	**10**	**5**	**20**	**31**	

Informing the Value Conversation

Identifying the Components of Patient-Centered Value

- What does patient-centered value mean to different communities?
- What elements, attributes, or components do different stakeholders consider to be part of value measurement?

Discussion

Questions for the Panel

- Do the direction, activities, and goals resonate with you?

- Will the activities meet the informational needs of your stakeholder community?

- What additional considerations should be kept in mind as we proceed?

BREAK (15 minutes)

We will return at 4:00 pm EST

pcori®

PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

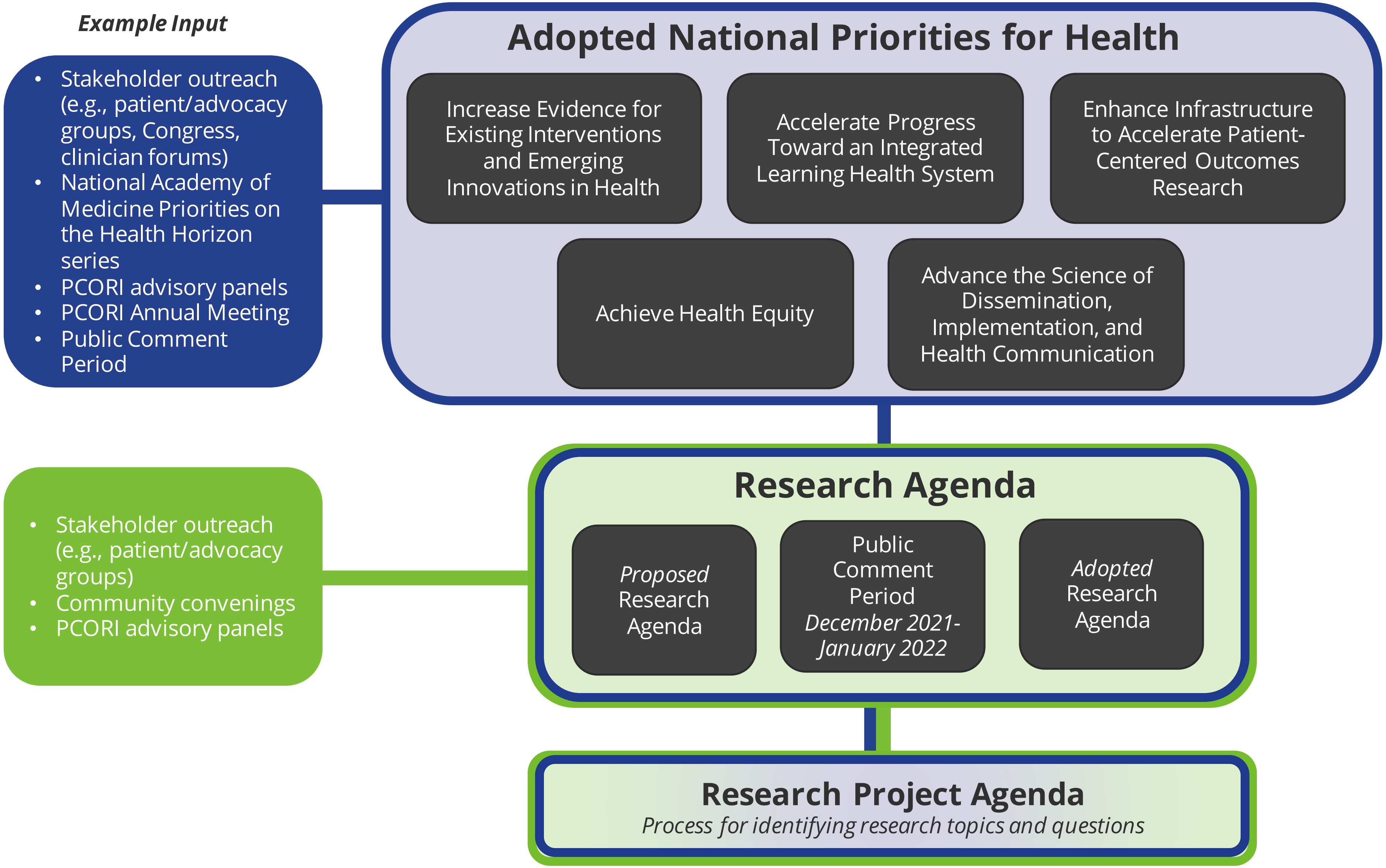
Update on Strategic Planning: PCORI’s Strategic Plan

Laura Lyman Rodriguez, PhD

Deputy Executive Director for

Strategy and Planning, Office of the Executive Director

Stakeholder and Public Input Inform Work



Example Input

- Stakeholder outreach (e.g., patient/advocacy groups, Congress, clinician forums)
- National Academy of Medicine Priorities on the Health Horizon series
- PCORI advisory panels
- PCORI Annual Meeting
- Public Comment Period

Adopted National Priorities for Health

Increase Evidence for Existing Interventions and Emerging Innovations in Health

Accelerate Progress Toward an Integrated Learning Health System

Enhance Infrastructure to Accelerate Patient-Centered Outcomes Research

Achieve Health Equity

Advance the Science of Dissemination, Implementation, and Health Communication

- Stakeholder outreach (e.g., patient/advocacy groups)
- Community convenings
- PCORI advisory panels

Research Agenda

Proposed Research Agenda

Public Comment Period *December 2021-January 2022*

Adopted Research Agenda

Research Project Agenda

Process for identifying research topics and questions

Adopted National Priorities for Health

Increase Evidence for Existing Interventions and Emerging Innovations in Health

Goal: Strengthen and expand ongoing comparative clinical effectiveness research focused on both existing interventions and emerging innovations to improve healthcare practice, health outcomes, and health equity.

Enhance Infrastructure to Accelerate Patient-Centered Outcomes Research

Goal: Enhance the infrastructure that facilitates patient-centered outcomes research to drive lasting improvements in health and transformation of both the research enterprise and care delivery.

Advance the Science of Dissemination, Implementation, and Health Communication

Goal: Advance the scientific evidence for and the practice of dissemination, implementation, and health communication to accelerate the effective sharing of comparative clinical effectiveness research results for public understanding and uptake into practice.

Achieve Health Equity

Goal: Expand stakeholder engagement, research, and dissemination approaches that lead to continued progress toward achieving health equity in the United States.

Accelerate Progress Toward an Integrated Learning Health System

Goal: Foster actionable, timely, place-based, and transformative improvements in patient-centered experiences, care provision, and ultimately improved health outcomes through collaborative, multisectoral research to support a health system that understands and serves the needs and preferences of individuals.

When We Last Spoke

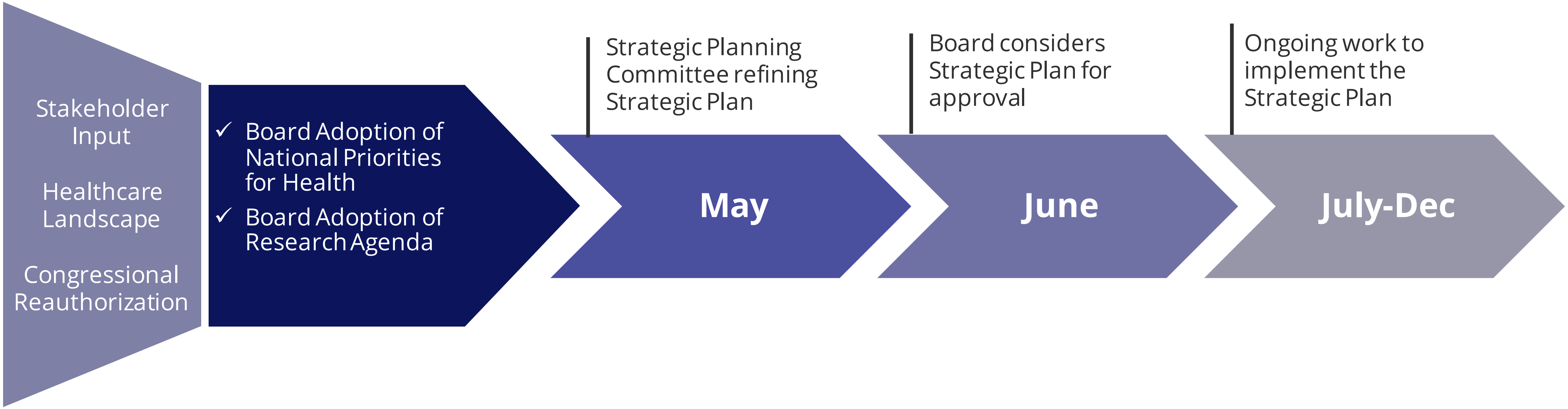
- This panel considered the proposed Research Agenda and how it could support a research portfolio to meet current and future needs.
- Panel members shared insights regarding PCORI's future work including the following:
  - The opportunity for PCORI's ongoing engagement work and the Research Agenda to support rare disease research, particularly through infrastructure development (e.g., workforce development and capacity building).
  - The importance of understanding that the rare disease community is an equity group and experiences disparities in care.
- This was integrated with input from other convenings, meetings, and discussions.

Adopted Research Agenda

Fund comparative clinical effectiveness research that

- Fills patient- and stakeholder-prioritized evidence gaps and is representative and inclusive of diverse and underrepresented patient populations and settings
- Advances the achievement of health equity and elimination of disparities with an emphasis on overcoming the effects on health and healthcare outcomes of racism, discrimination, and bias
- Builds the evidence base for emerging interventions by leveraging the full range of data resources and partnerships
- Examines the diverse burdens and clinical and economic impacts important to patients and other stakeholders
- Focuses on health promotion and illness prevention by addressing health drivers that occur where people live, work, learn, and play
- Integrates implementation science and advances approaches for communicating evidence so the public can access, understand, and act on research findings

Anticipated Timeline



Stakeholder Input

Healthcare Landscape

Congressional Reauthorization

- ✓ Board Adoption of National Priorities for Health
- ✓ Board Adoption of Research Agenda

May

Strategic Planning Committee refining Strategic Plan

June

Board considers Strategic Plan for approval

July-Dec

Ongoing work to implement the Strategic Plan

Developing a Comprehensive Approach

- Core Strategic Plan document
- Digital format (e.g., clickable, interactive webpages)
- Printable "at-a-glance" handouts (e.g., two-page PDFs)

Accessibility

- Demonstrate how PCORI achieves its mission
- Opportunity for stakeholders to see themselves in PCORI's vision
- Serve as a communication tool
- Serve as the basis for PCORI's next phase

Purpose

- Patients and caregivers
- Researchers and clinicians
- Congress and GAO
- Healthcare purchasers, payers, industry, hospitals and other health systems
- Broader health community

Audiences

Outline of Substantive Components in Strategic Plan

- **Introduction and A Bold Vision for PCORI's Second Decade**
  - Shares, briefly, PCORI's history and how we envision continuing our work such as the continued importance of diversity, equity, and inclusion and being responsive to an evolving health care and research landscape.
- **A Holistic Approach to Generating and Disseminating Patient- Centered CER**
  - Describes comprehensive set of activities (e.g., funding CER, engagement, D&I, infrastructure) that flow from the National Priorities for Health and facilitate the generation and dissemination of evidence to improve clinical decision making
  - Includes the National Priorities for Health and Research Agenda
- **Measuring Success and Monitoring Progress**
  - Provides general approach for assessing PCORI's progress towards the National Priorities for Health

Moving Towards Implementation Planning

- We are excited to *begin discussions* with Advisory Panels related to implementation planning. To start, we would like your input on strategic opportunities related to the Research Project Agenda (RPA).

- We are particularly interested in hearing about promoting an effective, transparent process for developing the RPA. Currently, this in a *transition* and we are considering opportunities to revise the process with an eye towards enhancing ability to be *nimble, adaptive, and responsive* to changes in the health environment.

- Aim for Research Project Agenda process that is:
  - Stakeholder-Driven
  - Responsive
  - Transparent
  - Systematic
  - Efficient

Anticipated Next Steps

Use feedback garnered from discussions to inform our process considerations along with actionable steps for implementation

Share update on process development with this panel at a future meeting

Beginning Discussions to Help Inform Implementation Planning

- What are meaningful ways to engage with stakeholder communities represented on this panel to solicit ideas and inform topic development (e.g., convenings, surveys, webinars)?
- What does a responsive, transparent Research Project Agenda development process look like from your perspective?
- Given the broad, integrated nature of the adopted National Priorities for Health and Research Agenda, how could PCORI demonstrate relevance to stakeholder communities represented by this panel?
- Based on this panel's expertise in CER for rare disease and engaging with the rare disease community, what are this panel's suggestions to stay up to date on emerging issues in this space?

Acknowledgments and Recap

Recognition of Departing Panel Members

Thank you!

We’d like to give special thanks to those members whose terms end this year:

- Doug Lindsay
- Saira Sultan

Panelist Recognition: Saira Sultan

- CEO, Connect4Strategies
- Saira Sultan has been at the nexus of patient groups, the medical community, drug/device manufacturers, and governmental organizations, for more than two decades.
- Sultan’s passion for rare and ultrarare disease issues culminated in her founding of the Haystack Project in 2016. Through Haystack, Sultan has given voice to the experiences and perspectives of patients with rare and ultrarare conditions to influence research and policy making.
- Contributions at RDAP meetings: Shared thoughts and recommendations on various topics including the process of applying for PCORI funding, promotion of funding announcements, the impact of telehealth on rare disease care during the COVID-19 pandemic, and the need for more engagement with patient groups.

Panelist Recognition: Doug Lindsay

- Co-Chair, PCORI Rare Disease Advisory Panel
- Speaker and Personal Medical Consultant, Founder – Doug Says LLC.
- Doug Lindsay's 14-year medical odyssey involved him working with faculty across different institutions, developing new uses for five existing prescription drugs, and developing the concept for two successful, innovative adrenal surgeries used to treat his case.
- Lindsay is a Community Advisory Board member for Washington University's Institute of Clinical and Translational Sciences and Institute for Public Health. He also provides personal medical consultant service to a small number of rare disease and complex condition patients who have found themselves trapped in the medical system.
- Contributions at the RDAP meetings: Shared thoughts and recommendation on several topics including developing guidance on applying for PCORI funding, use of social media to reach certain patient groups, the importance of rare disease community as an equity group, and the need for more research training for rare disease organizations.

Adjourn