Rare Diseases: PCORI's Portfolio, Resources, and Challenges

April 20, 2017

12:00-1:15 pm

PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

Before We Begin

- Thank you to those who submitted questions ahead of time, we will have open Q&A at the end of the webinar via the chat function on the right side of your screen
- We welcome your comments via Twitter to @PCORI and #PCORI
- This webinar will be archived on PCORI's site here http://www.pcori.org/get-involved/participate-pcori-events/pcori-practice

Speakers & Agenda

Jackie Gannon, Fellow, Eugene Washington PCORI Engagement Awards

Lia Hotchkiss, Director, Engagement Awards

Courtney Clyatt, Program Officer, Pipeline to Proposal

Parag Aggarwal, Senior Program Officer, Healthcare Delivery and Disparities Research

Topic	Time
Learning Objectives & PCORI Overview	12:00-12:05 pm
PCORI's Three Funding Programs • Eugene Washington PCORI Engagement Awards • Pipeline to Proposal Awards • Research Awards	12:05-12:35 pm
PCORI Resources for Rare Diseases	12:35-12:40 pm
Challenges and Questions from Registration Survey	12:40-12:45 pm
Open Q&A	12:45-1:10 pm
Adjourn	1:15 pm

Goals for Today's Webinar

- Increase your knowledge of various PCORI funding opportunities and provide examples of rare disease PCORI-funded projects
  - Eugene Washington PCORI Engagement Awards
  - Pipeline to Proposal (P2P) Awards
  - Research Awards
- Learn about resources with guidance on planning or executing a rare disease project
- Gain an understanding of common challenges faced by rare disease organizations when starting a project

PCORI: Mission and Strategic Goals

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

Our Strategic Goals:

- Increase quantity, quality, and timeliness of useful, trustworthy research information available to support health decisions
- Speed the implementation and use of patient-centered outcomes research evidence
- Influence research funded by others to be more patient-centered

We Fund Comparative Clinical Effectiveness Research (CER)

- Compares the effectiveness of two or more interventions with proven efficacy
- Answers questions that matter to patients and other clinical decision makers
- Measures benefits in real-world populations and is patient-centered
- Describes results in subgroups of people
- Helps consumers, clinicians, purchasers, and policy makers make informed decisions that will improve care for individuals and populations

Note: We do not fund cost-effectiveness research

We Fund Patient-Centered Outcomes Research (PCOR)

PCOR is a relatively new form of CER that…

- Considers patients' needs and preferences, and the outcomes most important to them
- Investigates what works, for whom, under what circumstances
- Helps patients and other healthcare stakeholders make better-informed decisions about health and healthcare options

Snapshot of PCORI-Funded Rare Disease Projects

Number of rare disease projects:

24 Engagement Awards
8 Pipeline to Proposals
24 Research Awards

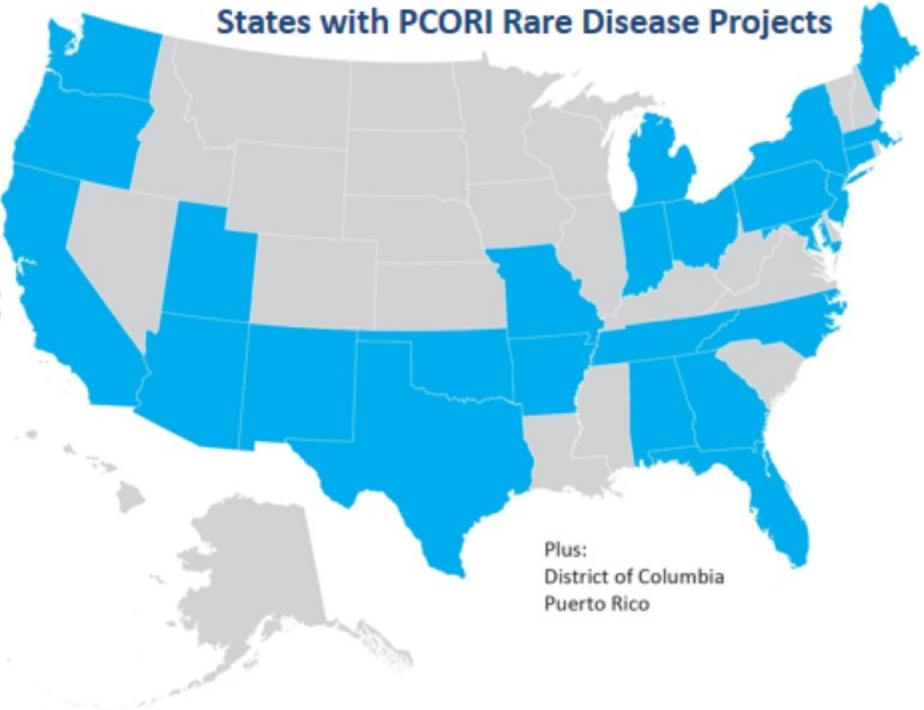
Amount awarded to rare diseases:

$3.9 million in Engagement Awards
$316,876 in P2P Awards
$61.2 million in Research

Number of states where we are funding rare disease research:

25 (plus the District of Columbia and Puerto Rico)

States with PCORI Rare Disease Projects



Plus:
District of Columbia
Puerto Rico

As of April 2017

Who Are Our Stakeholders?

- Payers
- Clinicians
- Caregivers/Family Members
- Purchasers
- Hospital/Health Systems
- Policy Makers
- Patients/Consumers
- Industry
- Patient/Caregiver Advocacy Organizations
- Training Institutions

Who Can Apply for PCORI Funding?

- Any private sector research organization

Nonprofit Organizations

For-Profit Organizations

- Any public sector research organization

Universities/ Colleges

Hospitals or Healthcare Systems

Local, State, or Federal Government

Laboratories

- Foreign Organizations or Nondomestic Components of Organizations based in US, **if clear benefit to US healthcare system**

NOTE

PI **must** be an employee of the prime applicant institution. Individuals are <u>not</u> eligible to submit research applications to PCORI.

Eugene Washington PCORI Engagement Awards

Building a PCOR Community Nationwide

Lia Hotchkiss, MPH

Director, Engagement Awards

Jackie Gannon, *National Urban Fellow*

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Program Overview

- A programmatic funding opportunity – not research awards
- Support projects that will build a community better able to participate in patient-centered research (PCOR) and comparative clinical effectiveness research (CER), as well as serve as channels to disseminate study results
- Projects will produce deliverables that are useful to awardees, PCORI, and the broader PCOR community for increasing patient and stakeholder engagement in PCOR and CER

Develop Community Skilled in PCOR

Involve Community in Research Processes

Engage Community in Dissemination

**As of April, 2017*

Types of Engagement Awards

- **Engagement Award (EA) projects**
  - Build **knowledge** base about how patients and other stakeholders want to participate in PCOR/CER or receive research findings;
  - Implement **training** or skill **development** initiatives to build capacity for engaging in PCOR; and
  - Strengthen channels for **disseminating** research findings.
- **Engagement Award Initiative Notice (EAIN)** supports **meetings/conferences** that align with PCORI's mission and strategic plan, and facilitate expansion of PCOR/CER in areas such as:
  - Research design and methodology
  - Research development
  - Dissemination and implementation

Awards of **up to $250,000** per project, up to **two years** in duration

Engagement Awards Portfolio Overview

Number of awards:

200*

Amount awarded:

$39.3 million

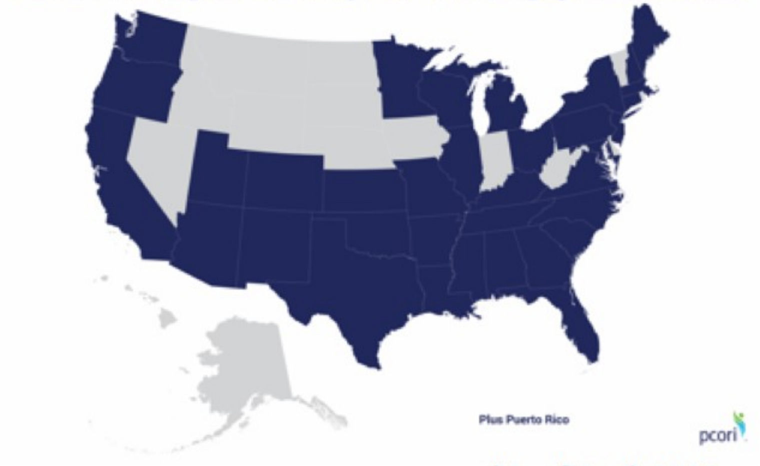
States with funded projects:

35 (plus DC and Puerto Rico)

24 are rare disease related

$3.9 million has gone toward funding rare disease projects

States with Eugene Washington PCORI Engagement Awards



As of April, 2017

Spotlight: PCORI-Funded Rare Disease Project

FOUNDATION FOR
PRADER-WILLI
RESEARCH
Working Toward an Independent Future

Foundation for Prader-Willi Research (FPWR):

Real-World Data Research Readiness: Engaging the Prader-Willi Syndrome (PWS) Community

- **Background**: Real-world data (RWD) is an emerging area of data acquisition and analysis, with the potential to transform clinical research and patient care. However, patient partnering in the development of RWD research and tools is lacking.
- **Project Outcome**: An in-person workshop and interactive web meeting to educate patient and stakeholder partners on real-world data and develop prioritized RWD research questions for PWS, while engaging PWS patient partners in the planning, conducting, and disseminating processes.
- **Project page**: http://www.pcori.org/research-results/2016/real-world-data-research-readiness-engaging-prader-willi-syndrome-community

Forming Relationships with Researchers/Biotech

FOUNDATION FOR
PRADER-WILLI
RESEARCH

- **Gathered the patients**
  - Accrued ~30 people through a Yahoo list serve in 2003
- **Connected to a biotech company**
  - They found out about a company testing a drug for PWS
  - FPWR provided them a report called, "**Evaluation of Pharmacologic Interventions in the Prader-Willi Syndrome Populations**: *Special Considerations*" that prompted the company to include a patient perspective in the drug development
  - FPWR helped by creating a research-ready patient community and facilitating patient recruitment through webinars, a registry, and social media using their pre-established network to spread the word
- **Stayed on top of other biotech/pharma drug development**
  - Suggested to a second company that their drug may be an effective PWS treatment and provided rationale for why
  - They ended up partnering to develop a successful Orphan Product Grant Program Application, which was funded by the FDA

Examples of Engagement Awards Projects:
Focused on Rare Disease Community

- **A Foundation for Building Strength:** ***Novel Stakeholder Engagement for Nemaline Myopathy, Patient-Centered Research***
  - Ongoing collaboration with stakeholder groups to further encourage patient-centered research, foster better patient-physician communication, and develop a path to treatment for the rare disease

- **University of Arkansas for Medical Sciences:** ***Developing and Testing Best Practices in Training for Academic/Community Research Partnerships***
  - Objective is to develop online training with usability and value to research partners, focusing on unique needs of rare and/or genetic condition patient-partner trainees

Conference Support Examples:
Focused on Rare Disease Community

- **Alstrom Syndrome International**: It's Time to Talk! It's Time to Listen!
- **Aplastic Anemia & MDS International Foundation**: Patient Advisory Committee for Clinical Trials
- **Cure CMD**: Building Momentum Through Congenital Muscular Dystrophy Stakeholder Participation
- **Foundation for Prader-Willi Research**: Real-World Data Research Readiness - Engaging the Prader-Willi Syndrome Community
- **Sturge-Weber Foundation**: The SWF Patient Engagement Conference
- **NEC Society**: Necrotizing Enterocolitis Symposium: A Transdisciplinary Approach to Improved NEC Outcomes
- **Phelan-McDermid Syndrome Foundation**: Phelan-McDermid Syndrome Patient-Centered Outcomes Workshop
- **PXE International**: Discovering What Matters Most
- **Sickle Cell Disease Association of America**: The National Sickle Cell Advocate Network
- **Vanderbilt University**: Tennessee Sickle Cell Disease Network

Engagement Awards NOT Intended to Support:

- Projects solely intended to improve patient engagement in healthcare service delivery,
- Projects intended to increase the number of patients who agree to be research subjects or participants
- Research studies
- Planning or pilot studies
- Projects designed solely to validate tools or instruments
- Delivery of health care
- Development of registries or recruitment of research or registry participants
- Development of decision support tools or clinical practice guidelines
- Meetings that don't focus on PCOR or CER
- Full-fledged projects to translate PCORI research findings into products and/or disseminate PCORI research results
- Projects proposed by PCORI-funded investigators to prepare for applying (i.e. bridge funding) to the Limited PCORI Funding Announcement: Dissemination and Implementation

How to Submit Applications

- http://www.pcori.org/funding-opportunities

Engagement Award: Knowledge, Training and Development, and Dissemination Awards

Key Deadlines	Type	Funds Available	Total Costs
LOI: June 1, 2017 by 5 p.m. ET **Application:** Full proposals are due 40 days after review and approval of the LOI.	Research Support Award		Award total costs may not exceed $250,000

June 1, 2017 Deadline

Engagement Award (EAIN): Research Meeting and Conference Support

Key Deadlines	Type	Funds Available	Total Costs
LOI: Not required **Application:** June 1, 2017 by 5 p.m. ET	Research Support Award		One-time award total costs must not exceed $50,000 and multi-year award total costs may not exceed $250,000.

June 1, 2017 Deadline (no LOI required)

Annual Funding Deadlines: **February 1, June 1, October 1

For More Information:

Engagement Awards Program

- Web Page: www.PCORI.org/eugene-washington-awards
- Email Address: ea@pcori.org
- Contact Number: 202-370-9312

Pipeline to Proposals Program

- Web Page: http://www.pcori.org/funding-opportunities/programmatic-funding/pipeline-proposal-awards
- Email Address: p2p@pcori.org

Pipeline to Proposal (P2P) Awards

Courtney Clyatt, MPH

Program Officer, Pipeline to Proposal

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Pipeline to Proposal Awards (P2P)

Mission

- The P2P program aims to build a national community of patient, stakeholder, and researcher partnerships that have the expertise and passion to participate in patient-centered outcomes research within their communities that leads to high-quality research.

- In addition, the P2P program is a funding mechanism to develop and strengthen the engagement in proposals submitted for funding.

Purpose

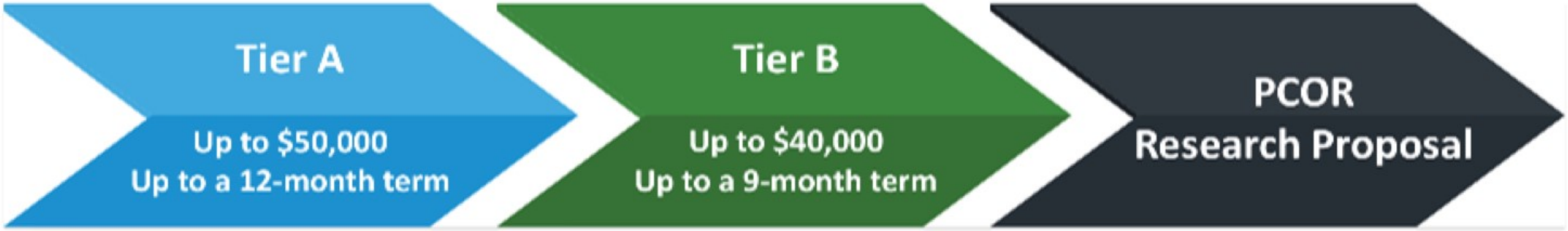
- Build capacity and cultivate the development of proposals with sound scientific rigor and **robust patient engagement**.

Overarching Goals of P2P

- Enabling the non-researcher community (including individual patients) to drive partnership development and research project (flip the funding)
- Developing research partnerships, infrastructure, and a diverse, skilled PCOR community, especially in underserved and underrepresented communities
- Creating a robust dissemination and implementation network that recognizes the PCORI brand
- Submission of high-quality PCOR/CER proposals to PCORI and other funders with strong engagement plans
- Learning about promising pre-engagement practices and methods in the P2P (P2P as a learning laboratory) and share with broader research community

New Two-Tiered Program

Projects participate in a two-tiered funding mechanism, which supports concentrated partnership development in Tier A and proposal development in Tier B. Each tier is designed to help patients and communities take another step toward producing community-led PCOR proposals.



Tier A

Up to $50,000
Up to a 12-month term

Tier B

Up to $40,000
Up to a 9-month term

PCOR Research Proposal

*P2P awards already in progress will continue to move through the old three-tiered program structure.

Examples of P2P Funded Rare Disease Projects

Stakeholder Engagement and Partnership Development (for $15,000) Building an Empowered Patient Community: Tackling Health Care for All Affected by **Mayer-Rokitansky-Küster-Hauser (MRKH) Syndrome**

Amy C. Lossie, PhD, with Beautiful You MRKH Foundation, Inc.

CER Question Development and Research Partnership Maturation (for $25,000) Bridging **Rare Disease** Patients and Data through Novel Research Partnerships, Indiana

Catherine Fairchild, JD, with Parkview Health

CER Proposal Development (for $50,000) Addressing Obesity in Latino Adolescents with **Spina Bifida**/Supporting Latino Families with Children with Spina Bifida

Ruth Bush with Spina Bifida San Diego

Rare Disease Research and Funding at PCORI

Parag Aggarwal, PhD

Senior Program Officer, Healthcare Delivery and Disparities Research Program

PCORI

April 20, 2017

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Presentation Overview

- PCORI Research Focus and Awards
- Rare Disease Research Portfolio and Example of Funded Study

PCORI's Research Focus and Awards

PCORI's Research Focus

Comparative Clinical Effectiveness Research

- Patient-centered
- Answering questions that matter to patients and other clinical decision makers
- Comparisons of outcomes that matter to patients
- Comparing the effectiveness of two or more interventions with proven efficacy

We Are Particularly Interested in Research That…

Focuses on high-priority conditions

- Affecting large numbers of people across a range of populations
- Placing a heavy burden on individuals, families, specific populations, and society
  - This includes many rare diseases

Our National Priorities for Research

Assessment of Prevention, Diagnosis, and Treatment Options

Improving Healthcare Systems

Communication & Dissemination Research

Addressing Disparities

CER Methods and Infrastructure

Funding Mechanisms

- Broad Funding Announcements
- Pragmatic Clinical Studies
- Targeted Funding Announcements

PCORI's Broad Funding Announcement

- Supports research on investigator-initiated research topics that address questions of importance to patients and other stakeholders.
- The announcement includes 5 different types of awards that align with PCORI's National Priorities for Research

Overview

Awards range from $750,000 to $5,000,000 in direct costs per project and are generally 3 years in duration

Pragmatic Clinical Studies

- Addresses critical evidence gaps, including topics of special interest to stakeholders, National Academy of Sciences, Agency for Healthcare Research and Quality
- Seeks to produce information that can be directly adopted by providers
- Often conducted in routine clinical settings
- Though often large, usually less complex protocols than traditional trials

Overview

Awards can be up to $10 million in direct costs per project and are generally 5 years in duration

Targeted Funding Announcements

- Seek research proposals on the highest-priority questions identified through PCORI's topic generation and research prioritization process
- Examples:
  - Clinical Management of Hepatitis C Infection
  - Treatment of Multiple Sclerosis
  - Management of Care Transitions for Emerging Adults with Sickle Cell Disease

Overview

Budget and project duration vary by funding announcement

http://www.pcori.org/research-results/how-we-select-research-topics/generation-and-prioritization-topics-funding-4

Help with Selecting a PCORI Funding Program

- A list of all open, closed, and upcoming funding announcements can be found here: http://www.pcori.org/funding-opportunities

- If you are not sure which is best for you, contact the PCORI Helpdesk:
  - Email: sciencequestions@pcori.org
  - Phone: (202) 627-1884
  - Online: http://www.pcori.org/PFA/inquiry

- **PCORnet**: PCORI's National Infrastructure
  - Clinical Data Research Network (rare disease cohorts)
  - Patient-Powered Research Network (rare disease-specific)
  - Archived Webinar: http://www.pcori.org/events/2017/pcornet-101
  - Contact: pmo@pcornet.org

Rare Disease Research Portfolio and Example of Funded Study

Rare Disease Portfolio

Overview

As of March 2017, PCORI has **23** active or completed comparative effectiveness research projects and **4** methods projects on rare disease, totaling **$61.2 million.**

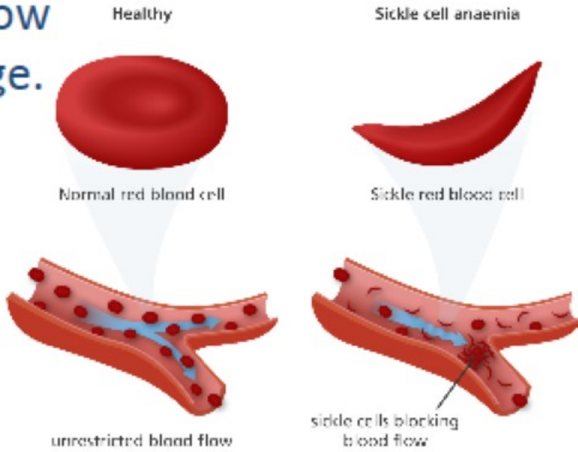
Rare Disease Portfolio

Specific conditions currently funded in our portfolio

- Acute myeloid leukemia
- Cerebral palsy
- Eosinophilic esophagitis
- Disorders of sex development
- Duarte galactosemia
- Hydrocephalus
- Lupus nephritis
- Chiari type I malformation (CM) and syringomyelia (SM)
- Polyarticular juvenile idiopathic arthritis
- Spinal cord injury and spina bifida
- Idiopathic subglottic stenosis Kawasaki disease
- Non-CF bronchiectasis
- Pediatric Crohn's disease
- Pediatric transverse myelitis
- Sickle cell disease
- Systemic scleroderma
- Urea cycle disorders

What Are the *Comparative Benefits and Drawbacks* of Pain Management Delivered *in Emergency Departments vs. in Dedicated Acute Care Facilities* for *Adults with Sickle Dell Disease*?

- **Background:**
  - Individuals with sickle cell disease have irregular hemoglobin in their RBCs that can cause the cells to change shape and stick to vessel walls, blocking the flow of blood. This causes pain and long-term organ damage.
  - There is no cure for SCD. Treatment involves management of pain symptoms.
  - Pain management is often delivered in emergency departments, but an alternative is infusion clinics.



- **Study Design:**
  - Observational study of adults with SCD comparing pain management delivered in EDs to pain management delivered in infusion clinics. Outcomes are the time to first dose of pain medication and time to discharge home.

- **Study Goal:** To change how the health system meets the needs of adults with SCD who are experiencing a sickle cell crisis.

Rare Disease-Specific PCORI Resources

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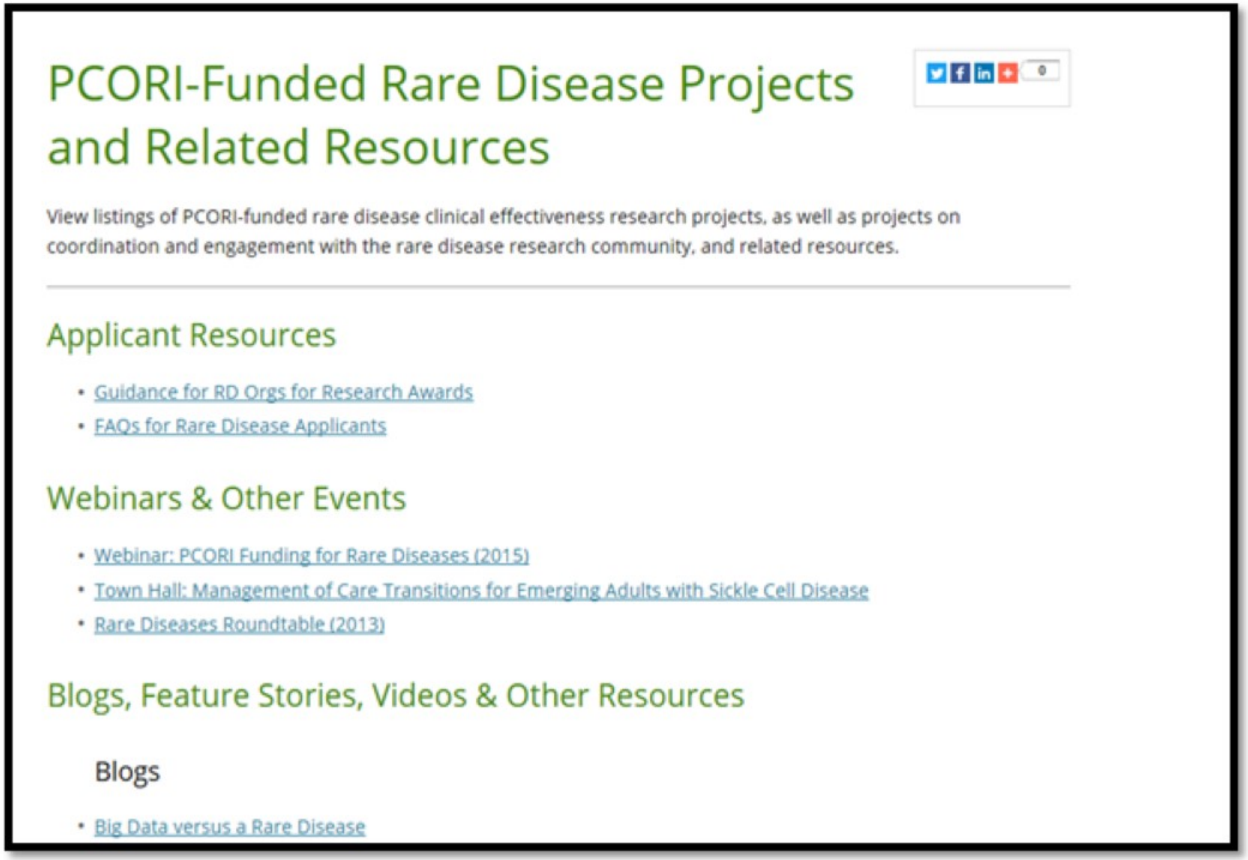
New Page for Rare Disease Resources



PCORI-Funded Rare Disease Projects and Related Resources

View listings of PCORI-funded rare disease clinical effectiveness research projects, as well as projects on coordination and engagement with the rare disease research community, and related resources.

Applicant Resources

- Guidance for RD Orgs for Research Awards
- FAQs for Rare Disease Applicants

Webinars & Other Events

- Webinar: PCORI Funding for Rare Diseases (2015)
- Town Hall: Management of Care Transitions for Emerging Adults with Sickle Cell Disease
- Rare Diseases Roundtable (2013)

Blogs, Feature Stories, Videos & Other Resources

Blogs

- Big Data versus a Rare Disease

Here you can find:

- All of PCORI's funded rare disease projects
- Applicant resources (rare disease-specific)
- Past webinars
- Rare disease PCORI-produced media, videos, and blogs

http://www.pcori.org/get-involved/join-advisory-panel/advisory-panel-rare-disease/pcori-funded-rare-disease-projects-and

Addressing Challenges and Questions from Pre-Registration Responses

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Open Q&A

- We welcome your questions and comments; use the chat function on the right side of your screen.
- This webinar will be archived here: http://www.pcori.org/get-involved/participate-pcori-events/pcori-practice

Follow-up Contact Information

Eugene Washington PCORI Engagement Awards: Lia Hotchkiss, *Director*, lhotchkiss@pcori.org
General inquiries or to schedule a program fit call: ea@pcori.org

Pipeline to Proposal: Courtney Clyatt, *Program Officer*, cclyatt@pcori.org
General inquiries:p2p@pcori.org

Research Awards: Parag Aggarwal, *Senior Program Officer* paggarwal@pcori.org
General inquiries: sciencequestions@pcori.org

Thank You for Attending!

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