PCORI Funding for Epilepsy Research

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ICARE: Interagency Collaborative to Advance Research in Epilepsy
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Patient-Centered Outcomes Research Institute

About PCORI

pcori.org

Our Mission

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

Pictured: PCORI Board of Governors (March 2012)

Our 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

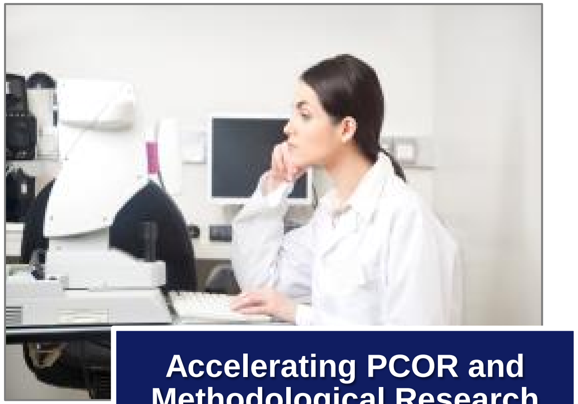
Our National Priorities for Research

Assessment of Prevention, Diagnosis, and Treatment Options

Improving Healthcare Systems

Communication & Dissemination Research

Addressing Disparities



Accelerating PCOR and Methodological Research

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We Engage Stakeholders at Every Step

Topic Selection and Research Prioritization

Merit Review

Study Design/ Implementation

Evaluation

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PCORI Funding for Epilepsy Research

The National Patient-Centered Clinical Research Network (PCORnet)

Clinical Data Research Networks

System-based networks, such as hospital systems

- 11 Networks
- $76.8 Million Awarded

Patient-Powered Research Networks

Patients with a single condition form a research network

- 18 Networks
- $16.8 Million Awarded

Coordinating Center

Provides technical and logistical assistance under the direction of the Steering Committee and PCORI Staff.

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Rare Epilepsy Network (REN) PPRN
Overview

- Principal Investigator: Janice Buelow, PhD, RN
- Lead Organization: Epilepsy Foundation
- Partnering Patient Organizations:
  - Aicardi Syndrome Foundation, Dravet Syndrome Foundation, Dup15q Alliance, Hope for Hypothalamic Hamartomas, Lennox-Gastaut Syndrome Foundation, Phelan-McDermid Syndrome Foundation, Tuberous Sclerosis Alliance

Rare Epilepsy Network (REN) PPRN, cont'd

Primary Goal

- To build a patient-centered and -driven database designed to provide the patients and their families an opportunity to participate in research that will improve lives and quality of care for people with rare epilepsies

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Rare Epilepsy Network (REN) PPRN, cont'd
Actions

- Build a secure data management system
  - Easy-to-navigate patient portal to support data collection directly from patients
  - Efficient questionnaire/instrument configuration
  - Real time data validations
  - Automated emailing of study participants, with periodic reminders, asking them to complete new question modules, or follow-up data collection;
  - The ability to import and export study data from and to other systems, such as the export of SAS files for analytical purposes, and the import of electronic health records.
  - The ability to import electronic medical record data collected through Blue Button into the REN database
  - The system will use encryption technologies and conform to 45 CFR 46 (human subjects protection) and HIPAA requirements.

Rare Epilepsy Network (REN) PPRN, cont'd

Actions

- Enroll 1500 patients
- Increase the diversity of patient population through partnerships with 47 affiliates throughout the US
- Develop policies for involving patients and caregivers in network governance
  - Caregiver initiated network with caregivers representing each partner organization
  - Develop patient and caregiver working groups to explore research and other issues most salient to patient
  - Leverage social media and innovative technologies for patient engagement

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Rare Epilepsy Network (REN) PPRN, cont’d

Actions

- Collect patient-generated information
  - Based on research questions that are of interest to REN members, a comprehensive set of questions will be asked of patient participants
  - Answers are collected in a standardized format
  - Leverage existing epilepsy-related common data element initiatives from National Institute of Neurological Disorders and Stroke (NINDS) and Centers for Disease Control (CDC)
- Harmonize a set of data domains to the PCORnet common data model

Cognitive AED Outcomes in Pediatric Localization Related Epilepsy (COPE)

Overview

- Principal Investigator: David W. Loring, PhD
- Organization: Emory University
- Sites:12 study sites throughout the United States.
- Goal:
  - Provide the information needed to help parents and their clinicians choose treatment options that maximize cognitive abilities in children with LRE and provide the data needed for practice guidelines to be established on the basis of cognitive side effect risks.
- PCORI Programmatic Area: Assessment of Prevention, Diagnosis, and Treatment Options

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Why Study Cognitive Effects of Drugs Used to Control Epilepsy?

- There are several available medications
- It is generally accepted that the medications have equivalent effects in controlling seizure rates
- Central nervous system toxicity is a major long-term problem with the medications
- There may be important differences in the level of CNS toxicity among the medications
- A study focusing on cognitive effects of the medications will fill the evidence gaps

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Cognitive AED Outcomes in Pediatric Localization Related Epilepsy (COPE)

Study Details

- Participants:
  - Children who are newly diagnosed with LRE by their treating physicians and are between 6 and 12 years
- Study Design:
  - Randomized Control Trial
- Treatment Arms:
  - 3 of the most common medications used to treat pediatric LRE: levetiracetam, lamotrigine, or oxcarbazepine
- Outcome of Interest:
  - Changes in cognitive abilities after exposure to medications
- Estimated Completion Date of Study:
  - Q2-3, 2016

Application and Review Process

pcori.org/apply

Annual Funding Cycles

Funding Cycle	Application Opens	Application Closes
Winter	November	February
Spring	February	May
Summer	May	August
Fall	August	November

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How to Submit an Application

Visit pcori.org/apply

- Key Dates
- Funding Announcements
- Application Guidelines and Templates
- Guidance on the PCORI Methodology Standards
- Sample Engagement Plans
- Frequently Asked Questions

PCORI’s Merit Review Process

Applications are reviewed against five criteria:

1. Impact of the condition on the health of individuals and populations
2. Potential for the study to improve healthcare and outcomes
3. Technical merit
4. Patient-centeredness
5. Patient and stakeholder engagement

- Applications are reviewed by a committee of two scientists, one patient, and one other stakeholder
- PCORI’s Board of Governors makes funding decisions based on merit review and staff recommendations

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Contact Us

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Thank You

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