pcornet® The National Patient-Centered Clinical Research Network

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PCORnet® Resources Are Surfacing Answers for Rare Diseases

PCORnet® is intended to improve the nation's capacity to efficiently conduct patient-centered health research, particularly comparative clinical effectiveness research (CER), by providing a large, highly representative network of health data, research expertise, and patient insights. PCORnet has been developed with funding from the Patient-Centered Outcomes Research Institute® (PCORI®).

SOLVING KEY CHALLENGES IN RESEARCH ON RARE DISEASES

Challenge: Small, geographically dispersed study populations

Solution: Real-world data from everyday healthcare encounters with more than 50 million people annually across the U.S.

Challenge: Lack of natural history data

Solution: Access to standardized longitudinal data

Challenge: Alignment of research questions and patient priorities

Solution: Patient and caregiver engagement drives meaningful research and adoption of actionable findings

CASE STUDIES: RARE DISEASES RESEARCH POWERED BY PCORnet®

Cognitive Behavioral Therapy and Real-Time Pain Management Intervention for Sickle Cell via Mobile Applications (CaRISMA)

Study Design: Intervention Trial

Funder: Patient-Centered Outcomes Research Institute® (PCORI®)

Principal Investigator: Charles Jonassaint, University of Pittsburgh

Research Question

Does mobile phone-delivered cognitive behavioral therapy (CBT) provide a greater benefit for daily pain and depression in adults with sickle cell disease (SCD) than digital education?

Using PCORnet, the study team

- Analyzed retrospective and prospective clinical data for 350 participants across 5 sites
- Conducted the largest behavioral intervention trial in SCD to date to compare the effectiveness of digital CBT and education to improve quality of life

Dissemination

Published results in *Blood Advances* and the *British Journal of Haematology*

Comparative Effectiveness of Epilepsy Surgery Versus Additional Anti-Seizure Medications for Lennox-Gastaut Syndrome

Study Design: Retrospective Observational Study

Funder: Patient-Centered Outcomes Research Institute® (PCORI®)

Principal Investigator: Sandi Lam, Lurie Children's Hospital

Research Question

Which type of treatment — more medicines or proceeding with surgery — will have the best outcomes for a child with Lennox-Gastaut Syndrome (LGS)?

Using PCORnet, the study team

- Developed computable phenotypes for LGS to analyze data from 4,000+ patients across 18 sites
- Is conducting the first study to directly compare the outcomes of using additional antiseizure medicine versus epilepsy surgery to treat LGS

Dissemination

- Published the study protocol in *Frontiers in Neurology*
- Published findings from surveys of caregivers in *Epilepsy and Behavior*

Utilizing PCORnet® to Support Transition from Pediatric to Adult Centered Care and Reduce Gaps in Recommended Care in Patients with Congenital Heart Disease (CHI-RON)

Study Design: Retrospective Observational

Funder: Patient-Centered Outcomes Research Institute® (PCORI®)

Principal Investigators: Thomas Carton, Louisiana Public Health Institute, and Anitha John, Children's National Research Institute

Research Questions

- How does receiving current recommended care affect long-term outcomes and healthcare needs among the numerous rare disease subtypes of congenital heart defects?
- What factors are associated with gaps in recommended care?
- Do patients report feeling better when they remain in specialty care?

Using PCORnet, the study team

- Linked patient-reported outcome data from a patient registry and electronic health record data accessible through PCORnet to assess study outcomes
- Leveraged the PCORnet® Common Data Model to develop an algorithm to tailor recruitment

Dissemination

- Published results in *JACC Advances*
- Published a paper on using PCORnet and patient engagement strategies to improve recruitment of research participants in *Medical Care*

Preserving Kidney Function in Children with Chronic Kidney Disease (PRESERVE)

Study Design: Retrospective Observational

Funder: Patient-Centered Outcomes Research Institute® (PCORI®)

Principal Investigators: Christopher Forrest and Michelle Denberg, The Children's Hospital of Philadelphia

Research Questions

- Which monitoring strategies and blood pressure medications best preserve kidney function in pediatric patients with chronic kidney disease (CKD)?
- What are the lived experiences of families and patients managing pediatric CKD?

Using PCORnet, the study team

- Included 20,100 children (ages 1–17) with CKD seen at 15 healthcare institutions between Jan. 2009-Dec. 2021
- Engaged caregivers to assess lived experiences managing blood pressure
- Expanded the PCORnet® Common Data Model for pediatric and rare kidney disease research

Dissemination

- Published findings on the comparative effectiveness of two treatments for high blood pressure in pediatric CKD in *JAMA Pediatrics*
- Published findings on using real-world electronic health record data to develop age-, sex-, and height-specific blood pressure percentiles for children in *eBioMedicine*

OTHER RARE DISEASES RESEARCH POWERED BY PCORnet®

- Biologic Abatement and Capturing Kids' Outcomes and Flare Frequency in Juvenile Spondyloarthritis (BACK-OFF JSpA)
- Neuroendocrine Tumors-Patient Reported Outcomes (NET-PRO)
- A Clinical Trial Readiness Study of Patient Reported Outcome Measures in Thrombotic Thrombocytopenic Purpura (THINK-TTP)
- Predictive Analytics via Networked Distributed Algorithms for Multi-System Diseases (PANDA-MSD)

POWER YOUR RARE DISEASE RESEARCH WITH PCORnet®

PCORnet resources are available to academic and industry researchers, patient advocacy groups, and other organizations from all funding sources and affiliations. PCORI also offers funding for broad pragmatic studies using PCORnet.

Contact the PCORnet® Front Door team to start a conversation about how you can use PCORnet to support your research.

PCORnet® is a national resource, funded by PCORI, that enables insights from high-quality health data, patient partnership, and research expertise to deliver fast, trustworthy answers that advance health outcomes. The network supports both observational and interventional research.

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