PCORI and PCORnet

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About PCORI
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An independent research institute authorized by Congress in 2010. Governed by a 21-member Board representing the entire healthcare community.

Funds comparative clinical effectiveness research (CER) that engages patients and other stakeholders throughout the research process.

Seeks answers to real-world questions about what works best for patients based on their circumstances and concerns.
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.
Research We Support
We Fund Research That…

- Studies the benefits and harms of interventions and strategies delivered in real-world settings
- Compares at least two alternative approaches
- Adheres to PCORI’s Methodology Standards
- Is based on health outcomes that are meaningful to the patient population
- Engages patients and other stakeholders at every stage
- Is likely to improve current clinical practices
We Require Patient-Centeredness and Patient and Stakeholder Engagement

Patient-Centeredness
- Does the project aim to answer questions or examine outcomes that matter to patients within the context of patient preferences?
- Research questions and outcomes should reflect what is important to patients and caregivers

Patient and Stakeholder Engagement
- Patients are partners in research, not just “subjects”
- Active and meaningful engagement between scientists, patients, and other stakeholders
- Community, patient, and caregiver involvement already in existence or a well-thought out plan
Supporting Engagement as a Tool to Advance Research
Engagement Goals

- Build a patient-centered outcomes research, or PCOR, community
- Engage the PCOR community in research
- Promote dissemination and implementation of PCOR research findings
Engagement as a Path To Useful, High-Quality Research

- Topic Selection and Research Prioritization
- Proposal Review; Design and Conduct of Research
- Dissemination and Implementation of Results
- Evaluation
PCORnet
Why did we establish PCORnet?

- National clinical research system is well-intentioned but flawed
- We’re not generating the evidence needed by patients and clinicians as they make decisions about their health care
- Health outcomes and disparities are not improving
- Current system is great except:
  - Too slow, too expensive, and not reliable
  - Doesn’t answer questions that matter most to patients
  - Unattractive to clinicians & administrators
  - *Doesn’t harness the true potential of health data*
Our Vision

PCORnet will enable rapid, large-scale, patient-centered clinical research in real-world care delivery systems and communities.

“Research Infrastructure Done Differently”
How is PCORnet different from other research networks?

**SCALE:** National Infrastructure for CER

**SIZE:** Includes large, diverse populations from real-world care settings

**STRUCTURE:** Engages patients & clinicians, systems in all aspects of research

**SCOPE:** Creates efficient & effective processes for all aspects of conducting research
Pivotal $100M Infrastructure Investment

11 Clinical Data Research Networks (CDRNs)
System-based networks, such as integrated delivery systems, academic medical centers, federally qualified health centers,

18 Patient-Powered Research Networks (PPRNs)
Patients with a condition in common form a research network, often in collaboration with academic researchers

Coordinating Center
Provides technical and logistical assistance under the direction of a steering committee and PCORI program staff

Complementary and synergistic capabilities in the two types of networks
29 CDRN and PPRN awards were approved on December 17th by PCORI’s Board of Governors.

This map depicts the number of PCORI funded Patient-Powered or Clinical Data Research Networks that have coverage in each state.
Diverse Contributors + Rich Data = High Potential

- Patient-Powered Registries
- Academic Health Centers
- Health Information Exchanges
- Integrated Care Delivery Systems
- Federally Qualified Health Centers
- Clinical & Translational Science Awardees
- Data from Payers (e.g., CMS)
- Pharmacy Data Vendors
- Disease Advocacy Groups

PCORI
PCORnet Organizational Structure

PCORnet STEERING COMMITTEE

Members represent:
- Each Clinical Data Research Network
- Each Patient-Powered Research Network
- Patients
- HHS agencies:
  - NIH
  - FDA
  - AHRQ
  - CDC
  - CMS
  - ONC
  - ASPE
- Medical product and device manufacturers
- PCORnet and Coordinating Center

PCORnet Executive Committee

PCORI

COORDINATING CENTER

11 CLINICAL DATA RESEARCH NETWORKS

18 PATIENT-POWERED RESEARCH NETWORKS

PROJECT MANAGEMENT OFFICE

TASK FORCES

- GOVERNANCE
- DATA PRIVACY
- ETHICS & REGULATORY
- DATA STANDARDS & SECURITY
- HEALTH SYSTEMS INTERACTIONS
- PATIENT & CONSUMER ENGAGEMENT
- PATIENT GENERATED OUTCOMES
- CLINICAL TRIALS
- RARE DISEASES
- BIOREPOSITORIES
- OBESITY
Winter 2015: Coming Into View

The world’s first network infrastructure to:

- Be based primarily on EHR data, rather than claims data
- Support both large observational studies and embedded randomized clinical trials
- Involve patients, clinicians, and health systems leaders in governance and use of the network
Coming Into View – Funded PCORnet Demonstration Projects

- **Aspirin for Secondary Prevention** – an RCT comparing two doses of aspirin in patients with CAD

- **CER in the Weight Cohort** – one or two large observational studies

- **Rapid-Cycle Research** with health systems and health plans – multi-system comparative research on systems improvement
Coming Into View – A National Resource

- Able to conduct simple queries rapidly and repeatedly
- Able to conduct large observational studies affordably using a common data model, distributed querying, sharing of data when needed
- Able to conduct clinical trials affordably through improved contracting, IRB coordination, engagement of clinicians and sites, and identification, recruitment, consenting and follow-up of subjects.
- Openness to data linkage with other databases (e.g., registries, CMS) for funded studies
- Openness to collaboration with outside investigators across a wide range of topics
Key Progress to Date for the CDRNS

- Governance structure in place with patient representation
- Mapping data to Version 1 of PCORnet Common Data Model
- Exploring approaches to obtaining complete data on their established population (across time and place)
- Establishing obesity cohort and other disease cohorts
- Collaborating on protocol for first interventional trial
- Streamlining approaches to IRB approval
- Exploring approaches for engaging clinicians in pragmatic clinical trials
- Collaborating and sharing best practices across PCORnet
Key Progress to Date for the PPRNS

- Patients involved in PPRN governance
- Progress in prioritizing research questions
- Plans for launching the patient portals and recruiting patient members
- Obtaining IRB approvals
- Exploring approaches to collecting data through leveraging EHR data, initiating patient surveys and transforming registry data
- Transforming data into the CDM
- Discussing ideas for collaborative studies (cross-PPRN and CDRN-PPRN projects)
Update on Governance Policies

- Decision-making and Leadership policy has been approved by the PCORnet Steering Committee
- What is a PCORnet study and data policies currently under review
- “External investigators” and “Authorship” policies under development
- All policies should be voted on by the PCORnet Steering Committee by Spring 2015
- Policies will enter an “active learning phase” for 6 months thereafter and be revised as necessary
Early Opportunity to Prove the Concept

PCORI has identified a unique early opportunity to support an *interventional individual-level clinical trial* that will inform future research studies in PCORnet.

The trial will be characterized by *operational simplicity* and *clinical relevance*.

Topics were *generated by the PCORnet community*, with multi-stakeholder prioritization process.
Expectations for the Aspirin Dosing Trial

- PCORI plans to provide up to $10 million in funding for direct costs for up to three years.
- Evaluate and refine PCORnet’s data and clinical trials infrastructure.
- Investigators must use efficient recruitment strategies to ensure broad representation from the patient community in the trial.
- Trial will follow 1000’s of patients in order to determine whether there are clinically meaningful differences in the effectiveness and safety of two commonly prescribed doses of aspirin.
- Trial must engage patients, clinicians, and health system leaders throughout the research process to capture relevant patient-centered outcomes from real-world clinical settings and optimize data collection across networks.
Capacities will be in place to support all three types of research: Interventional Trials, Observational Studies, and Rapid Cycle Healthcare Delivery Research.

PCORnet Infrastructure and the “Research Triple Aim”

Longer Term: Platform for Variety of Study Designs

- Early planning underway for observational studies using cohorts of patients who are obese or overweight.
- **VERY** Early planning underway on demonstration projects of interest to health system leaders.
Find Us Online

www.pcori.org
# 71 Participating Health Systems in 11 CDRNs…

<table>
<thead>
<tr>
<th>Lead Organization (Network name)</th>
<th>Partnered Health Systems</th>
</tr>
</thead>
</table>
| **Patient Outcomes Research To Advance Learning (“PORTAL”)** | 1. Denver Health  
2. Group Health Cooperative  
3. Group Health Research Institute  
4. HealthPartners Research Foundation  
5. KP Center for Effectiveness & Safety Research  
6. KP Colorado (Institute for Health Research)  
7. KP Georgia (Center for Health Research)  
8. KP Hawaii (Center for Health Research)  
9. KP Mid-Atlantic States (Mid-Atlantic Permanente Research Institute)  
10. KP Northern California (Division of Research)  
11. KP Northwest (Center for Health Research)  
12. KP Southern California (Department of Research & Evaluation) |
| **PaTH: Towards a Learning Health System in the Mid-Atlantic Region (“PaTH”)** | 1. University of Pittsburgh/UPMC and UPMC Health Plan  
2. Penn State College of Medicine/Hershey Medical Center  
3. Temple University School of Medicine/Temple Health  
4. Johns Hopkins Univ/Johns Hopkins Health System & Johns Hopkins Health Care |
| **Children’s Hospital of Philadelphia (“PEDSNet”)**       | 1. Children’s Hospital of Philadelphia  
2. Cincinnati Children’s Hospital Medical Center  
3. Children’s Hospital Colorado  
4. Nemours Children’s Health System  
5. Nationwide Children’s Hospital  
6. St. Louis Children’s Hospital  
7. Seattle Children’s Hospital  
8. Boston Children’s Hospital |
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| Harvard Medical School ("SCILHS") | 1. Boston Children’s Hospital  
2. Partners (Mass General and Brigham and Women’s Hospital)  
3. Wake Forest Baptist University Medical Center  
4. Cincinnati Children’s Hospital  
5. University of Texas Health Science Center  
6. Columbia University  
7. Morehouse School of Medicine  
8. University of Mississippi Medical Center |
| Vanderbilt University ("Mid-South CDRN") | 1. Vanderbilt Health System  
2. Vanderbilt Healthcare Affiliated Network (VHAN)  
3. Greenway Medical Technologies |
| University of California San Diego ("pSCANNER") | 1. University of California Research eXchange (UC-ReX) network  
2. VA Informatics and Computing Infrastructure (VINCI)  
3. UC San Diego (UCSD),  
4. VA Tennessee Valley Healthcare System |
| University of Kansas Medical Center ("Great Plains Collaborative") | 1. University of Kansas Medical Center  
2. Children’s Mercy Hospital  
3. University of Wisconsin-Madison  
4. Medical College of Wisconsin  
5. Marshfield Clinic  
6. University of Health Sciences at San Antonio  
7. University of Texas Southwestern Medical Center  
8. University of Iowa Healthcare  
9. University of Minnesota  
10. University of Nebraska |
### Network name

**Lead Organization/PI**

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<tr>
<th>Network name</th>
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</table>
| NYC-CDRN     | 1. New York-Presbyterian Hospital  
               2. Weill Cornell Medical College  
               3. Columbia University Medical School  
               4. Montefiore Medical Center  
               5. NYU Langone Medical Center  
               6. Mount Sinai Health System  
               7. Clinical Directors Network (FQHC) |
| Chicago Community Trust (“CAPriCORN”) | 1. Loyola University  
                                           2. Northwestern Medicine  
                                           3. Northshore University Health System  
                                           4. University of Chicago  
                                           5. University of Illinois Hospital & Health Sciences System  
                                           6. Cook County Health and Hospital System  
                                           7. Alliance of Chicago’s Federally Qualified Health Centers  
                                           8. Hines VA  
                                           9. Jesse Brown VA  
                                           10. Lurie Children’s Hospital  
                                           11. Children’s Hospital of University of Illinois  
                                           12. Comer Children’s Hospital |
| Accelerating Data Value Across a National Community Health Center Network (“ADVANCE”) | 1. OCHIN  
                                               2. Health Choice Network  
                                               3. Fenway Clinic |
| Louisiana Public Health Institute (“Louisiana Clinical Data Research Network – LACDRN”) | 1. Greater New Orleans Health Information Exchange  
                                                                            2. Louisiana State University  
                                                                            3. Louisiana Public Health Institute  
                                                                            4. Tulane University |
80 Different Organizations Involved in 18 PPRNs...

<table>
<thead>
<tr>
<th>Organization</th>
<th>PI</th>
<th>Condition</th>
<th>Population Size</th>
</tr>
</thead>
<tbody>
<tr>
<td>Duke University</td>
<td>Laura Schanberg</td>
<td>Juvenile Rheumatic Disease</td>
<td>9000</td>
</tr>
<tr>
<td>ALD Connect, Inc</td>
<td>Florian Eichler</td>
<td>Adrenoleukodystrophy</td>
<td>3000</td>
</tr>
<tr>
<td>Phelan-McDermid Syndrome Foundation</td>
<td>Megan O'Boyle</td>
<td>Phelan-McDermid Syndrome</td>
<td>737</td>
</tr>
<tr>
<td>Immune Deficiency Foundation</td>
<td>Kathleen Sullivan</td>
<td>Primary Immunodeficiency Diseases</td>
<td>1250</td>
</tr>
<tr>
<td>University of Pennsylvania</td>
<td>Peter Merkel</td>
<td>Vasculitis</td>
<td>500 (Pilot)</td>
</tr>
<tr>
<td>Parent Project Muscular Dystrophy</td>
<td>Holly Peay</td>
<td>Duchenne and Becker muscular dystrophy</td>
<td>4000</td>
</tr>
<tr>
<td>Arbor Research Collaborative for Health</td>
<td>Bruce Robinson</td>
<td>Primary Nephrotic Syndrome (Focal Segmental Glomerulosclerosis [FSGS], Minimal Change Disease [MCD], and Membranous Nephropathy [MN] Multiple Sclerosis</td>
<td>1250</td>
</tr>
<tr>
<td>Epilepsy Foundation</td>
<td>Janice Beulow</td>
<td>Aicardi Syndrome, Lennox-Gastaut Syndrome, Phelan-McDermid Syndrome, Hypothalamic Hamartoma, Dravet Syndrome, and Tuberous Sclerosis</td>
<td>1500</td>
</tr>
<tr>
<td>Genetic Alliance, Inc</td>
<td>Sharon Terry</td>
<td>Alström syndrome, Dyskeratosis congenital, Gaucher disease, Hepatitis, Inflammatory breast cancer, Joubert syndrome, Klinefelter syndrome and associated conditions, Metachromatic leukodystrophy, Pseudoxanthoma elasticum (PXE), Psoriasis</td>
<td>50- 50,000</td>
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<tr>
<td>University of California, San Francisco</td>
<td>Mark Pletcher</td>
<td>Cardiovascular Health</td>
<td>100,000</td>
</tr>
<tr>
<td>Cincinnati Children’s Hospital Medical Center</td>
<td>Peter Margolis</td>
<td>Pediatric Crohn’s Disease and Ulcerative Colitis</td>
<td>15,000</td>
</tr>
<tr>
<td>Crohn’s &amp; Colitis Foundation of America</td>
<td>R. Balfour Sartor</td>
<td>Inflammatory Bowel Disease (Crohn’s disease and ulcerative colitis)</td>
<td>30,000</td>
</tr>
<tr>
<td>Global Healthy Living Foundation</td>
<td>Seth Ginsberg</td>
<td>Arthritis (rheumatoid arthritis, spondyloarthritis), musculoskeletal disorders (osteoporosis), and inflammatory conditions (psoriasis)</td>
<td>50,000</td>
</tr>
<tr>
<td>American Sleep Apnea Association</td>
<td>Susan Redline</td>
<td>Sleep Apnea</td>
<td>50,000</td>
</tr>
<tr>
<td>COPD Foundation</td>
<td>Richard Mularski</td>
<td>COPD</td>
<td>50,000</td>
</tr>
<tr>
<td>Accelerated Cure Project for Multiple Sclerosis</td>
<td>Robert McBurney</td>
<td>Multiple Sclerosis</td>
<td>20,000</td>
</tr>
<tr>
<td>University of South Florida</td>
<td>Rebecca Sutphen</td>
<td>Hereditary Breast and Ovarian Cancer (HBOC)</td>
<td>17,000</td>
</tr>
<tr>
<td>Massachusetts General Hospital</td>
<td>Andrew Nierenberg</td>
<td>Major Depressive Disorder, Bipolar Disorder</td>
<td>50,000</td>
</tr>
</tbody>
</table>