Launching PCORnet, the National Patient-Centered Clinical Research Network

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PCORnet
PCORnet: the National Patient-Centered Clinical Research Network

The goal of PCORI’s National Patient-Centered Clinical Research Network Program is to improve the nation’s capacity to conduct CER efficiently, by creating a large, highly representative, national patient-centered clinical research network for conducting clinical outcomes research.

The vision is to support a learning US healthcare system, which would allow for large-scale research to be conducted with enhanced accuracy and efficiency.
Patient-centeredness is at the heart of PCORI’s mission and vision

- The Patient-Centered Outcomes Research Institute (PCORI) is an independent, non-profit health research organization authorized by the Patient Protection and Affordable Care Act of 2010.
- PCORI funds patient-centered research to assist patients, caregivers, and other stakeholders in making informed health decisions.

**Mission**
PCORI helps people 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.

**Vision**
Patients and the public have the information they need to make decisions that reflect their desired health outcomes.
Patients and stakeholders play critical roles in all aspects of PCORI’s work.
Overall objectives of PCORnet: achieving a single functional research network

- **Engagement** of patients, providers and health system leaders
- Support and conduct of multi-center observational and interventional CER studies
- External data and research partners participate with PCORI-funded networks
- Researchers not directly affiliated with PCORnet participate through collaborative arrangements.
- PCORnet partners use the resources created with PCORI’s support for a range of activities supported by other organizations.
29 CDRN and PPRN awards were approved on December 17th by PCORI’s Board of Governors.
Clinical Data Research Network Partners
Goals for Clinical Data Research Networks (CDRNs)

- Extracts from **EHR data** are standardized within PCORnet
- A minimum of **1 million patients** enrolled
- Capable of implementing **clinical trials**
- Individual network’s data in **interoperable format** with other awardee networks and successfully responds to **queries**
- **Patients, health system leaders, and clinicians** involved in governance and use of network
- At least **three patient cohorts** identified, characterized, and surveyed
CDRN Highlights

• Networks of academic medical centers, hospitals and physician practices
• Networks of non-profit integrated health systems
• Networks of low income clinics
• Networks leveraging AHRQ investments and NIH investments (CTSAs)
• Inclusion of Health Information Exchanges
• Wide geographical spread
• Inclusion of underserved populations
• Range from 1M covered lives to 28M
# 11 CDRNs

<table>
<thead>
<tr>
<th>CDRN Name</th>
<th>Lead Organization</th>
<th>Principal Investigator</th>
</tr>
</thead>
<tbody>
<tr>
<td>ADVANCE</td>
<td>Oregon Community Health Information Network</td>
<td>Jennifer DeVoe</td>
</tr>
<tr>
<td>CAPriCORN</td>
<td>The Chicago Community Trust</td>
<td>Terry Mazany</td>
</tr>
<tr>
<td>Great Plains Collaborative</td>
<td>University of Kansas Medical Center</td>
<td>Lemuel Waitman</td>
</tr>
<tr>
<td>Louisiana Clinical Data</td>
<td>Louisiana Public Health Institute</td>
<td>Anjum Khurshid</td>
</tr>
<tr>
<td>Research Network</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Mid-South CDRN</td>
<td>Vanderbilt University</td>
<td>Russell Rothman</td>
</tr>
<tr>
<td>NYC-CDRN</td>
<td>Weill Medical College of Cornell University</td>
<td>Rainu Kaushal</td>
</tr>
<tr>
<td>PEDSNet</td>
<td>The Children’s Hospital of Philadelphia</td>
<td>Christopher Forrest</td>
</tr>
<tr>
<td>PORTAL</td>
<td>Kaiser Foundation Research Institute</td>
<td>Elizabeth McGlynn</td>
</tr>
<tr>
<td>pSCANNER</td>
<td>University of California, San Diego</td>
<td>Lucila Ohno-Machado</td>
</tr>
<tr>
<td>P2ATH</td>
<td>University of Pittsburgh</td>
<td>Rachel Hess</td>
</tr>
<tr>
<td>SCIHLS</td>
<td>Harvard University</td>
<td>Kenneth Mandl</td>
</tr>
</tbody>
</table>
# CDRNs Disease Cohorts

<table>
<thead>
<tr>
<th>Organization</th>
<th>Common Cohort</th>
<th>Rare Cohort</th>
</tr>
</thead>
<tbody>
<tr>
<td>ADVANCE</td>
<td>Diabetes</td>
<td>Co-infection with HIV and hepatitis C virus</td>
</tr>
<tr>
<td>CAPriCORN</td>
<td>Anemia; Asthma</td>
<td>Sickle cell disease; Recurrent C. Difficile colitis</td>
</tr>
<tr>
<td>Great Plains Collaborative</td>
<td>Breast Cancer</td>
<td>Amyotrophic Lateral Sclerosis (ALS)</td>
</tr>
<tr>
<td>Louisiana Clinical Data Research Network</td>
<td>Diabetes</td>
<td>Sickle Cell Disease, Rare Cancers</td>
</tr>
<tr>
<td>NYC-CDRN</td>
<td>Diabetes</td>
<td>Cystic fibrosis</td>
</tr>
<tr>
<td>Mid-South CDRN</td>
<td>Coronary Heart Disease (CHD)</td>
<td>Sickle Cell Disease (SCD)</td>
</tr>
<tr>
<td>PEDSNet</td>
<td>Inflammatory bowel disease</td>
<td>Hypoplastic left heart syndrome</td>
</tr>
<tr>
<td>PORTAL</td>
<td>Colorectal Cancer</td>
<td>Severe Congenital Heart Disease</td>
</tr>
<tr>
<td>pSCANNER</td>
<td>Congestive Heart Failure</td>
<td>Kawasaki Disease</td>
</tr>
<tr>
<td>P2ATH</td>
<td>Atrial Fibrillation</td>
<td>Idiopathic Pulmonary Fibrosis</td>
</tr>
<tr>
<td>SCIHLS</td>
<td>Osteoarthritis</td>
<td>Pulmonary arterial hypertension</td>
</tr>
</tbody>
</table>
Patient Powered Research Network Partners
Goals for Patient Powered Research Networks (PPRNs)

- Target size of **0.5% of U.S population** with condition: (> 50 patients for rarest diseases; 50,000 for most common)
- **Patient-reported data** collected for at least 80% of cohort
- **Patients** involved in governance
- **Standardized data** suitable for sharing with other infrastructure members and successfully responds to queries
PPRN Highlights

• Variety of **stakeholders** in participating organizations and in leadership team: patients, advocacy groups, physician organizations, academic centers, PBRNs etc.
• Strong understanding of **patient engagement**
• Significant range of **conditions and diseases**
• Variety in **populations** represented (including pediatrics, underserved populations etc.)
• 50% **rare diseases**
• Significant range in the **maturity** of the group in terms of data available
• Several have capacity to work with **biospecimens**
## 9 PPRNs in “non rare” conditions

<table>
<thead>
<tr>
<th>Organization</th>
<th>PI</th>
<th>Condition</th>
<th>Proposed PPRN Population Size</th>
</tr>
</thead>
<tbody>
<tr>
<td>Accelerated Cure Project for Multiple Sclerosis</td>
<td>Robert McBurney</td>
<td>Multiple Sclerosis</td>
<td>20,000</td>
</tr>
<tr>
<td>American Sleep Apnea Association</td>
<td>Susan Redline</td>
<td>Sleep Apnea</td>
<td>50,000</td>
</tr>
<tr>
<td>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>COPD Foundation</td>
<td>Richard Mularski</td>
<td>Chronic Obstructive Pulmonary Disease</td>
<td>50,000</td>
</tr>
<tr>
<td>Crohn’s and 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), osteoporosis, and inflammatory conditions (psoriasis)</td>
<td>50,000</td>
</tr>
<tr>
<td>Massachusetts General Hospital</td>
<td>Andrew Nierenberg</td>
<td>Major Depressive Disorder (MDD) and Bipolar Disorder (BP)</td>
<td>50,000</td>
</tr>
<tr>
<td>Univ of California, San Francisco</td>
<td>Mark Pletcher</td>
<td>Cardiovascular health</td>
<td>100,000</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>
</tbody>
</table>
## 9 PPRNs in rare conditions

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<tr>
<th>Organization</th>
<th>PI</th>
<th>Condition</th>
<th>Proposed PPRN Population Size</th>
</tr>
</thead>
<tbody>
<tr>
<td>ALD Connect, Inc</td>
<td>Florian Eichler</td>
<td>Adrenoleukodystrophy</td>
<td>3,000</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>1,250</td>
</tr>
<tr>
<td>Duke University</td>
<td>Laura Schanberg</td>
<td>Juvenile Rheumatic Disease</td>
<td>9,000</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>1,500</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>
</tr>
<tr>
<td>Immune Deficiency Foundation</td>
<td>Kathleen Sullivan</td>
<td>Primary Immunodeficiency Diseases</td>
<td>1,250</td>
</tr>
<tr>
<td>Parent Project Muscular Dystrophy</td>
<td>Holly Peay</td>
<td>Duchenne and Becker muscular dystrophy</td>
<td>4,000</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>University of Pennsylvania</td>
<td>Peter Merkel</td>
<td>Vasculitis</td>
<td>500</td>
</tr>
</tbody>
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PCORnet Steering Committee

- Each Clinical Data Research Network
- Each Patient Powered Research Network
- HHS agencies
  - NIH
  - FDA
  - AHRQ
  - CDC
  - CMS
  - ONC
  - ASPE
- Medical product / device manufacturers
- PCORI and Coordinating Center
Coordinating Center Co-Directors

Richard Platt, MD, MSc  
Robert M. Califf, MD
CC Executive Leadership Committee

- **Richard Platt,** Harvard Pilgrim Health Care Institute, Harvard Medical School
- **Robert Califf,** Director, Duke Translational Medicine Institute, Vice Chancellor for Clinical and Translational Research
- **Eric Larson,** Group Health Research Institute
- **Mark McClellan,** Brookings Institution
- **Erin Holve,** AcademyHealth
- **Bray Patrick-Lake,** Director of Stakeholder Management, Duke Translational Medicine Institute
Task Forces
Governance & Collaboration Task Force

Leads: Rich Platt, Erin Holve, CDRN/PPRN co-lead TBD

Goal/Purpose:
Develop policies that support trust and collaboration
Data Standards, Security & Network Infrastructure Task Force

Leads: Jeff Brown, Lesley Curtis, CDRN/PPRN co-lead TBD

Goal/Purpose:
Create the PCORnet Distributed Research Network that facilitates multi-site research across the CDRNs, PPRNs, and others
1. User creates and submits query (a computer program)

2. Individual CDRNs/PPRNs retrieve query

3. CDRNs/PPRNs review and run query against their local data

4. CDRNs/PPRNs review results

5. CDRNs/PPRNs return results via secure network

6. Results are aggregated
PCORnet DRN Operations Center

- Reporting
- Project Management
- Policies and Procedures
- Query Fulfillment
- ETC...

PCORnet DRN Secure Portal

Knowledge Management System
Cross project lessons learned, query tracking, meta-data capture, search functions, etc

Projects
- Utilization trends
- Observational studies
- Pragmatic and clinical trials

Analytic Tools
- Modular programs
- Summary tables
- Query interface
- Menu-driven query
- Data checking tools
- Reporting tools

Administration
- Security \ Access control
- File \ Query repository
- User administration
- Workflow management

CDRN 1
- Data Partner A
- Data Partner B
- Data Partner C
- Data Partner D

CDRN 2
- Data Partner A
- Data Partner B
- Data Partner C
- Data Partner D

CDRN 11
- Data Partner A
- Data Partner B
- Data Partner C
- Data Partner D

PPRN 1
PPRN 2
PPRN 18
Other data resources
Multiple Networks Sharing Infrastructure

Each organization can participate in multiple networks

Each network controls its governance and coordination

Networks share infrastructure, data curation, analytics, lessons, security, software development
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Data Privacy Task Force

Lead: Deven McGraw, CDRN/PPRN co-lead TBD

Goal/Purpose:
• Identify privacy issues raising particular challenges
• Highlight promising or best practices for addressing them
• Develop privacy policies to govern data sharing
Patient & Consumer Engagement Task Force

Lead: Sean Tunis, Pat Deverka, CDRN/PPRN co-lead TBD

Goal/Purpose:

- Ensure engagement of patients and consumers in all components of PCORnet
- Serve as technical resource, with PCORI staff
Health Systems Interactions Task Force

 Leads: Eric Larson, Katherine Newton, CDRN/PPRN co-lead TBD

Goal/Purpose:
Help the CDRNs create a supportive environment for clinical research with their clinicians and clinical leadership
Ethics and Regulatory Task Force

Leads: Jeremy Sugarman, Joseph Ali, Rob Califf, CDRN/PPRN co-lead TBD

Goal/Purpose:
Address ethical and regulatory issues related to research that arise in PCORnet’s work
Patient-Reported Outcomes Task Force

Lead: Amy Abernethy, CDRN/PPRN co-lead TBD

Goal/Purpose: Focus on measurement, collection, and analysis of patient-generated information
Clinical Trials Task Force

Lead: Rob Califf, CDRN/PPRN co-lead TBD

Goal/Purpose:
• Adopt methods, standards, and quality by design principles for clinical trials
• Develop pathways for trials, oversee trial conduct, feed back learnings
PCORnet Initial Interventional Trial

Step 1
• Topic Generation

Step 2
• CDRNs/PPRNs Rank Topics Using PCORI's Research Prioritization Criteria

Step 3
• PCORI Program Development Committee & PCORI Board of Governors Approve Topic

Step 4
• PCORI issues Funding Announcement
Rare Diseases Task Force

 Leads: Priya Kishnani, Rachel Richesson, CDRN/PPRN co-lead TBD

Goal/Purpose:

• Support identification of populations and research priorities for studies of rare diseases
• Create an information source for rare diseases research
• Create a discussion and advocacy forum to identify and advocate for needs specific to rare diseases research
Biorepositories Task Force

Lead: Kristin Newby, CDRN/PPRN co-lead TBD

Goal/Purpose:
Support a regulatory-compliant, comprehensive, and sustainable Network-wide biorepository to serve PCORnet research
Obesity Task Force

Lead: Matthew Gillman, CDRN/PPRN co-lead TBD

Goal/Purpose: Facilitate construction of the obesity cohort at each CDRN, and identify potential research uses
Some takeaways

We need a new national capability for efficient, large-scale interventional and observational patient centered research.

PCORnet will need to:

- Establish priorities that clinicians, clinical leaders, patients, and investigators share.
- Facilitate trust leading to collaboration between networks.
- Embed research into practice settings without disrupting clinical operations.
- Create a distributed data network that protects patients’ confidential information.
- Develop oversight procedures that protect patients while minimizing redundancy.
- Engage individuals and organizations beyond the initial awardees.