

# PCORI and PCORnet: Creating a Research Framework to Engage Patients

A PART OF THE *FASTERCURES* VALUE AND COVERAGE ISSUE BRIEF SERIES

*This issue brief, the sixth in a series prepared by Breakaway Policy Strategies for FasterCures, provides an overview of the new Patient-Centered Outcomes Research Institute and its recently established Patient-Centered Research Network, outlining potential implications for patient-centered research, care delivery, and value.*

## Background

The Patient-Centered Outcomes Research Institute (PCORI), authorized by Congress under the Affordable Care Act (ACA), is an independent, nonprofit health research organization dedicated to creating tools that strengthen evidence-based decisions about healthcare treatment options. The organization frames patient-centered outcomes research as focusing on the characteristics, conditions, preferences, and desired outcomes of individual patients. PCORI is led by a 21-member board of governors, representing a broad spectrum of expertise and viewpoints in health research.

PCORI's mission is to "help people make informed health care decisions, and improve 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."<sup>1</sup> As of July 2014, the organization has awarded 280 grants funding comparative effectiveness research (CER) across five national priority areas:

- 1) assessing prevention, diagnosis, and treatment options,
- 2) improving healthcare systems,
- 3) communication and dissemination research,
- 4) addressing disparities, and
- 5) accelerating patient-centered outcomes research and methodological research.

The patient perspective is an essential component across these priority areas. It is a unique focus that has distinguished PCORI in the field of health services and outcomes research.

To help advance patient-centered research, PCORI recently established the Patient-Centered Outcomes Research Network (PCORnet), which will build the infrastructure for a new national network of patient data for CER.

## Why PCORnet?

*The Problem:* Randomized clinical trial design is generally considered to be the gold standard of scientific research. However, several issues potentially limit the value of current clinical research for patients and providers in real-world healthcare practice settings:

- Patients often lack the means to obtain or properly interpret research data specific to their particular conditions, treatment options, and concerns;
- Clinical research requires extensive resources that are not always available for smaller health systems, organizations, and patient groups in need; and
- The cost and lengthy nature of clinical research may leave patient questions unanswered and providers with incomplete information.

<sup>1</sup> "Mission and Vision." Patient-Centered Outcomes Research Institute. <http://www.pcori.org/about-us/mission-and-vision/>.

*PCORnet Solution:* PCORnet aims to advance the speed, effectiveness, and affordability of clinical research. This research better supports a learning healthcare system,<sup>2</sup> in which clinical research and high-quality evidence is integrated with patient care and treatment decision-making in an interactive process that builds a body of evidence.

The aim of the initiative is to enhance both the development and application of the nation's clinical research system through promoting:

- 1) **Patient Involvement:** Engaging patients in the process of issue prioritization, research, design, analysis, and dissemination of results.
- 2) **Evidence-Based Decision Making:** Providing broader access to robust CER and data on healthcare treatment strategies that help patients, providers, and health systems make more informed decisions.
- 3) **Real-World Application:** Providing pragmatic information relevant to patient concerns more quickly than currently employed clinical research processes.

## PCORnet: The New National Patient Data Network

PCORnet is a national, patient-centered clinical research network built to incorporate interoperable networks of data on millions of patients, drawn from electronic health records (EHRs), insurance claims, and patient-reported information. The network is structured to feature direct involvement from patients, providers, and health systems, and respond to their particular research needs. It also contains high levels of stakeholder representation and input, including from patient advocacy groups; medical product, pharmaceutical, and device manufacturers; and Department of Health and Human Services (HHS) agencies—such as the National Institutes of Health (NIH), the Food and Drug Administration (FDA), the Centers for Medicare and Medicaid Services (CMS), and the Agency for Healthcare Research and Quality (AHRQ).

### PCORnet Structure

There are currently 11 Clinical Data Research Networks (CDRN) and 18 Patient-Powered Research Networks (PPRN) within PCORnet, outlined in detail below. In addition, there is a Coordinating Center to help manage

technical and logistical support across the PCORnet data networks.

The initiative is overseen by a steering committee, composed of representatives from each CDRN and PPRN; patient advocacy groups; medical product, pharmaceutical, and device manufacturers; and multiple government agencies, including the NIH, FDA, Centers for Disease Control and Prevention (CDC), CMS, and AHRQ.

## Clinical Data Research Networks

Each CDRN must be a partnership between two or more health systems that have access to EHRs for at least 1 million patients. The current CDRNs (outlined in Table 1) range from 1 million to 28 million patients, and include partnerships among academic medical centers, hospitals, physician practices, nonprofit health systems, and low-income community-based clinics. The CDRNs are regional entities, with some networks encompassing large university, city, or statewide pacts.

Each CDRN is responsible for collecting and structuring patient data in an interoperable format for both a prevalent condition among large populations and a rare condition among smaller, but still sufficiently sized, populations. All patient data culled from the CDRNs will be standardized within PCORnet.

## Patient-Powered Research Networks

Whereas CDRNs are run by health systems, the PPRNs are governed by patients and patient advocacy groups, with a focus on patient-centered research for a specific condition. PPRNs also include participation from a variety of health stakeholders, including physician organizations, hospital groups, and academic health centers.

Each network has chosen a different disease focus, with half concentrating on prevalent conditions and half on rare disorders. The current PPRNs (outlined in Tables 2 and 3) must collect patient-reported data for 0.5 percent of the U.S. population afflicted with the network's disease focus, defined as at least 50,000 patients for prevalent conditions and 50 patients for rare ones. Similar to CDRNs, PPRNs will share patient data in a standardized format within PCORnet.

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<sup>2</sup> For more information on a learning healthcare system, see: Etheredge, Lynn. "Health Affairs." *Rapid Learning: A Breakthrough*

*Agenda.* Health Affairs, 1 July 2014.  
<http://content.healthaffairs.org/content/33/7/1155.abstract?=&right>

**Table 1: Clinical Data Research Networks, 2014**

Network	Organization	State	Prevalent Condition	Rare Condition	Populations Covered
<a href="#">Accelerating Data Value Across a National Community Health Center Network (ADVANCE)</a>	Oregon Community Health Information Network	Oregon	Diabetes	Co-infection with HIV and Hepatitis C virus	Underserved, rural, urban, rare disorders, children, minority, other vulnerable populations
<a href="#">Chicago Area Patient Centered Outcomes Research Network (CAPriCORN)</a>	The Chicago Community Trust	Illinois	Anemia, Asthma	Sickle cell disease, recurrent <i>C. difficile colitis</i>	Underserved, urban, rare disorders, children, geriatric, minority
<a href="#">Greater Plains Collaborative (GPC)</a>	University of Kansas Medical Center	Kansas	Breast Cancer	Amyotrophic Lateral Sclerosis	Underserved, rural, urban, rare disorders, children, geriatric, minority, disabled, other vulnerable populations
<a href="#">Kaiser Permanente &amp; Strategic Partners Patient Outcomes Research To Advance Learning (PORTAL) Network</a>	Kaiser Foundation Research Institute	California	Colorectal Cancer	Severe Congenital Heart Disease	Underserved, rural, urban, rare disorders, children, geriatric, minority, disabled
<a href="#">Louisiana CDRN (LACDRN)</a>	Louisiana Public Health Institute	Louisiana	Diabetes	Sickle Cell Disease; Rare Cancers	Underserved, rural, urban, rare disorders, children, geriatric, minority, disabled, other vulnerable populations
<a href="#">Mid-South CDRN</a>	Vanderbilt University	Tennessee	Coronary Heart Disease	Sickle Cell Disease	Underserved, rural, urban, rare disorders, children, geriatric, minority, disabled, other vulnerable populations
<a href="#">National Pediatric Learning Health System (PEDSNet)</a>	The Children's Hospital of Philadelphia	Pennsylvania	Inflammatory bowel disease	Hypoplastic left heart syndrome	Underserved, urban, rare disorders, children, minority
<a href="#">New York City Clinical Data Research Network (NYC-CDRN)</a>	Weill Medical College of Cornell University	New York	Diabetes	Cystic fibrosis	Underserved, urban, rare disorders, children, geriatric, minority, disabled, other vulnerable populations
<a href="#">Patient-oriented SCALable National Network for Effectiveness Research (pSCANNER)</a>	University of California, San Diego (UCSD)	California	Congestive Heart Failure	Kawasaki Disease	Underserved, urban, rare disorders, children, geriatric, minority, disabled
<a href="#">PaTH: Towards a Learning Health System in the Mid-Atlantic Region</a>	University of Pittsburgh	Pennsylvania	Atrial Fibrillation	Idiopathic Pulmonary Fibrosis	Underserved, rural, urban, rare disorders, children, minority, other vulnerable populations
<a href="#">Scalable Collaborative Infrastructure for a Learning Healthcare System (SCILHS)</a>	Harvard University	Massachusetts	Osteoarthritis	Pulmonary arterial hypertension	Underserved, rural, urban, rare disorders, children, geriatric, minority, disabled, other vulnerable populations

Source: The National Patient-Centered Clinical Research Network, "Clinical Data Research Networks"

**Table 2: Patient-Powered Research Networks, 2014****Prevalent Conditions**

Network	Organization	State	Clinical condition	Proposed Population
<a href="#">American BRCA Outcomes and Utilization of Testing Patient-Powered Research Network (ABOUT Network)</a>	University of South Florida	Florida	Hereditary breast and ovarian cancer	17,000
<a href="#">ARthritis patient Partnership with comparative Effectiveness Researchers (AR-PoWER PPRN)</a>	Global Healthy Living Foundation	New York	Arthritis (rheumatoid arthritis, spondyloarthritis), musculoskeletal disorders (osteoporosis), and inflammatory conditions (psoriasis)	50,000
<a href="#">CCFA Partners Patient Powered Research Network</a>	Crohn's and Colitis Foundation of America	New York	Inflammatory bowel disease (Crohn's disease and ulcerative colitis)	30,000
<a href="#">COPD Patient Powered Research Network</a>	COPD Foundation	Florida	Chronic Obstructive Pulmonary Disease	100,000
<a href="#">Health eHeart Alliance</a>	University of California, San Francisco	California	Cardiovascular health	100,000
<a href="#">ImproveCareNow: A Learning Health System for Children with Crohn's Disease and Ulcerative Colitis</a>	Cincinnati Children's Hospital Medical Center	Ohio	Pediatric Crohn's disease and ulcerative colitis	15,000
<a href="#">Mood Patient-Powered Research Network</a>	Massachusetts General Hospital	Massachusetts	Major Depressive Disorder and Bipolar Disorder	50,000
<a href="#">Multiple Sclerosis Patient-Powered Research Network</a>	Accelerated Cure Project for Multiple Sclerosis	Massachusetts	Multiple Sclerosis	20,000
<a href="#">Sleep Apnea Patient Centered Outcomes Network (SAPCON)</a>	American Sleep Apnea Association	District of Columbia	Sleep Apnea	50,000

Source: *The National Patient-Centered Clinical Research Network, "Patient-Powered Research Networks"*

## PCORnet: Impacts

### Patient Engagement: Getting Involved

Patient engagement in research is a primary goal and a unique focus for PCORI and PCORnet. Patients and patient advocates are [encouraged to become involved](#) in many, if not all, aspects of the institute's work, including: submitting research questions, reviewing PCORI funding applications, participating in research workshops and other events, serving in advisory panels, and providing public comments.

### Value of Care

PCORnet was initiated in January 2014. The networks are in early stages—addressing data standardization, governance, methods, and the establishment of robust infrastructure for patient-centered clinical research. As the initiative progresses further, PCORnet may be a game-changer for patient-centered outcomes and effectiveness research, potentially helping to equip patients and their providers with the information needed to make better decisions and reach desired health outcomes while helping to constrain spending. The scope of research and geographic span of the national network could substantially improve the quality, production, and accessibility of reliable healthcare research.

**Table 3: Patient-Powered Research Networks, 2014****Rare Conditions**

Network	Organization	State	Clinical condition	Proposed Population
<a href="#">ALD Connect</a>	ALD Connect, Inc.	Massachusetts	Adrenoleukodystrophy	3,000
<a href="#">Community-Engaged Network for All (CENA)</a>	Genetic Alliance, Inc.	District of Columbia	Alström syndrome, Dyskeratosis congenital, Gaucher disease, Hepatitis, Inflammatory breast cancer, Joubert syndrome, Klinefelter syndrome and associated conditions, Metachromatic leukodystrophy, Pseudoxanthoma elasticum (PXE), Psoriasis	50-50,000
<a href="#">DuchenneConnect Patient-Report Registry Infrastructure Project</a>	Parent Project Muscular Dystrophy	New Jersey	Duchenne and Becker muscular dystrophy	4,000
<a href="#">NephCure Kidney Network for Patients with Nephrotic Syndrome</a>	Arbor Research Collaborative for Health	Michigan	Primary Nephrotic Syndrome (Focal Segmental Glomerulosclerosis [FSGS], Minimal Change Disease [MCD], and Membranous Nephropathy [MN] Multiple Sclerosis	1,250
<a href="#">Patients, Advocates and Rheumatology Teams Network for Research and Service (PARTNERS) Consortium</a>	Duke University	North Carolina	Juvenile Rheumatic Disease	9,000
<a href="#">Phelan-McDermid Syndrome Data Network</a>	Phelan-McDermid Syndrome Foundation	Florida	Phelan-McDermid Syndrome	737
<a href="#">PI Patient Research Connection: PI-CONNECT</a>	Immune Deficiency Foundation	Maryland	Primary Immunodeficiency Diseases	1,250
<a href="#">Rare Epilepsy Network (REN)</a>	Epilepsy Foundation	Maryland	Aicardi Syndrome, Lennox-Gastaut Syndrome, Phelan-McDermid Syndrome, Hypothalamic Hamartoma, Dravet Syndrome, and Tuberous Sclerosis	1,500
<a href="#">Vasculitis Patient Powered Research Network</a>	University of Pennsylvania	Pennsylvania	Vasculitis	500

Source: *The National Patient-Centered Clinical Research Network, "Patient-Powered Research Networks"*

**Looking Ahead**

PCORnet is an innovative and challenging initiative. Data interoperability, privacy, ethics, and research methods require careful attention in order for the project to be successful. In addition, funding for PCORI (provided through a per capita annual fee on all health plans) expires after Fiscal Year 2019 and will have to be reauthorized by Congress. Patients, patient advocates,

and providers should closely follow how the PCORI and PCORnet initiatives develop.

Although the present focus of the network is primarily on the effectiveness of current interventions, the PCORnet national infrastructure may help accelerate innovation in healthcare research and lead to the identification of new and optimal cures in the long-term. Advocates for new treatments and cures will be interested to see if or when PCORnet's clinical networks move into experimental design with the potential for faster, larger, and less costly clinical trials.



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