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Patient-powered research networks: building capacity for conducting patient-centered clinical outcomes research

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ABSTRACT

The Patient-Centered Outcomes Research Institute (PCORI) recently launched PCORnet to establish a single inter-operable multicenter data research network that will support observational research and randomized clinical trials. This paper provides an overview of the patient-powered research networks (PPRNs), networks of patient organizations focused on a particular health condition that are interested in sharing health information and engaging in research. PPRNs will build on their foundation of trust within the patient communities and draw on their expertise, working with participants to identify true patient-centered outcomes and direct a patient-centered research agenda. The PPRNs will overcome common challenges including enrolling a diverse and representative patient population; engaging patients in governance; designing the data infrastructure; sharing data securely while protecting privacy; prioritizing research questions; scaling small networks into a larger network; and identifying pathways to sustainability. PCORnet will be the first distributed research network to bring PCOR to national scale.

INTRODUCTION

The Patient-Centered Outcomes Research Institute (PCORI)^{1–3} recently launched an ambitious new resource known as PCORnet, the National Patient-Centered Clinical Research Network to increase speed, efficiency, and relevance of clinical research in the USA.⁴ In support of this initiative, PCORI awarded \$93.5 million to support 29 health research networks (11 clinical data research networks (CDRNs) and 18 patient-powered research networks (PPRNs)) that together will become a large, interoperable, highly representative, national ‘network of networks’ for integrating patient-generated data and electronic health information, and conducting comparative effectiveness research (CER).

PCORnet is a novel distributed data network that includes substantial roles and responsibilities for patients and their caregivers in building network capacity, governing and using the health data, and directing a patient-centered research agenda. By encouraging and embedding patients in leadership roles, PCORnet realigns the focus of the existing clinical research enterprise from investigator-driven to patient-centered, thereby advancing the PCORI vision of a paradigm shift that powerfully expands the currently limited roles of the patients and their caregivers in clinical research participation and decision-making. The PPRNs, networks operated

and governed by patients, advocacy organizations, and their clinical research partners, will be leading this shift within PCORnet and driving change into our current translational research paradigm. This paper provides a brief overview of the nascent PPRN ecosystem.

EIGHTEEN PPRNS

In December 2013, PCORI awarded funding to 18 PPRNs that represent patients with a diverse set of diseases and conditions (table 1). Half of the networks represent patients with rare diseases. The 18 PPRN abstracts are available at <http://pcornet.org/patient-powered-research-networks/>.

PCORI has several goals for the PPRNs to accomplish by the end of the 18-month funding period. The PPRNs will:

- Enroll $\geq 0.5\%$ of the US population with the specified condition in their network, a minimum of 50 000 patients for most common conditions (less for patients with rare disorders).
- Develop a governance structure and operating policies that fully involves the participants and families, and fosters relationships with researchers to generate and prioritize research questions important to the network community’s membership.
- Collect patient-generated health information that is suitable for research from $\geq 80\%$ of its membership.
- Standardize data suitable for sharing with other PCORnet networks.

To achieve these ambitious goals, PPRNs will build on the strengths of their respective network, including (1) a primary focus on improving the health outcomes of individuals and their families, (2) a strong foundation of cooperation, contribution, and trust among diverse stakeholders, and (3) a high degree of participation by the participant community to identify true patient-centered outcomes and direct the research agenda. PPRNs will bring the participant voice not only to their respective networks, but to PCORnet as a whole. The PPRN skills and expertise in engaging participants will serve as a powerful facilitator for robust infrastructure development. We anticipate a variety of challenges to implementation, however, some of which are common within PCORnet and some of which will be unique to individual PPRNs.

ENROLLING A DIVERSE AND REPRESENTATIVE MEMBERSHIP

PPRNs aim to recruit participants with demographic and clinical characteristics sufficient to

Table 1 Eighteen Patient-Powered Research Networks (PPRNs), PCORnet, December 2013

Network*	Principal investigator	Organization	Disease or condition	Rare disease	Type of organizations in the PPRN†			
					Group of patients T1	Network by researchers T2	Online communities T3	Patient registries T4
ALD Connect	Florian Eichler	Massachusetts General Hospital	X-linked adrenoleukodystrophy	●	●	●		
American BRCA Outcomes and Utilization of Testing Patient-Powered Research Network (ABOUT Network)	Rebecca Sutphen	University of South Florida Morsani College of Medicine	Hereditary breast and ovarian cancer		●	●	●	●
Arthritis Patient Partnership with Comparative Effectiveness Researchers (AR-poWER PPRN)	Seth Ginsberg	Global Healthy Living Foundation	Inflammatory arthritis		●		●	
CCFA Partners Patient Powered Research Network	R. Balfour Sartor/ Michael Kappelman	Crohn's and Colitis Foundation of America	Inflammatory bowel disease		●	●	●	●
Community Engaged Network for All (CENA)	Sharon Terry	Genetic Alliance	10 disease network‡	●	●			●
ImproveCareNow: A Learning Health System for Children with Crohn's Disease or Ulcerative Colitis	Peter Margolis	ImproveCareNow Network	Inflammatory bowel disease		●	●		●
Mood Patient-Powered Research Network	Andrew Nierenberg	Massachusetts General Hospital	Mood disorders		●	●		
Multiple Sclerosis Patient Powered Research Network	Robert McBurney	Accelerated Cure Project for MS	Multiple sclerosis		●			●
NephCure Kidney Network for Patients with Nephrotic Syndrome	Bruce Robinson	Arbor Research Collaborative for Health	Primary nephrotic syndrome	●	●	●		
Patient Research Connection: PI-Connect	Kathleen Sullivan	Immune Deficiency Foundation/ USIDNET	Primary immunodeficiency	●	●	●		
Patients, Advocates and Rheumatology Teams Network for Research and Service (PARTNERS) Consortium	Laura Schanberg/Esi Morgan Dewitt/Marc Natter	Duke University Medical Center/ PARTNERS	Pediatric rheumatic disease	●	●	●	●	●
Phelan-McDermid Syndrome Data Network	Megan O'Boyle/Lyz Horn/Paul Avillach	Phelan-McDermid Syndrome Foundation/Harvard Medical School	Phelan-McDermid syndrome	●	●		●	●
Rare Epilepsy Network (REN)	Janice Buelow	Epilepsy Foundation	Catastrophic rare epilepsies	●				
Sleep-Apnea-Patient Centered OutcomesNetwork (SA-PCON)	Susan Redline	Brigham and Women's Hospital	Sleep apnea		●	●		
The COPD Patient Powered Research Network	John Walsh/Richard Mularski	The COPD Foundation & Kaiser- The Center for Health Research	Chronic obstructive pulmonary disease		●	●		
The DuchenneConnect Patient-Report Registry Infrastructure Project	Holly Peay	Parent Project Muscular Dystrophy	Duchenne and Becker muscular dystrophies	●	●		●	●
The Health eHeart Alliance	Mark Pletcher	University of California, SF	Cardiovascular health			●	●	
The Vasculitis Patient Powered Research Network	Peter Merkel	University of Pennsylvania	Vasculitis	●	●	●		

T1 US-based network, group, or organization of patients (either with a physical or virtual presence) of any size that has as a central goal of the establishment and growth of an activated cadre of individuals to provide their own patient-reported data for PCOR.

T2 Networks or groups that have been developed in part through the efforts of clinicians, researchers, or delivery systems to participate in comparative effectiveness research, including randomized clinical trials. These are networks or groups that were not initiated or managed by patients and/or family members.

T3 Networks or groups of patients that have been convened through the efforts of internet-based or social media-based vendors, such as online communities, groups convened to use personal health records, or specifically for purposes of participating in research.

T4 Existing patient registries in which member patients are active in the governance of registry activities and management of the registry data. These registries were initiated by patients and/or family members.

*Abstracts for each PPRN can be found in the online JAMA supplement for this manuscript or at <http://pcornet.org/patient-powered-research-networks/>.

†Each project can be built from multiple organizations.

‡Alström syndrome, dyskeratosis congenita, Gaucher disease, hepatitis, inflammatory breast cancer, Joubert syndrome, Klinefelter syndrome and associated sex chromosome conditions, metachromatic leukodystrophy, pseudoxanthoma elasticum, and psoriasis.

represent the range of expression of the conditions, and the lived experience of affected individuals and their families. PPRNs are developing strategies to reach historically underrepresented groups defined in terms of race/ethnicity, socioeconomic status, geographic location, and health literacy, as well as address the needs of patients across a wide range of clinical severity. While each PPRN has a unique approach, strategies to enhance the diversity and the representativeness of their communities include working with community or faith-based groups to engage underserved patients, developing language and literacy level appropriate recruitment and risk/benefit materials (print, video, audio, electronic), and engaging communities in traditionally underserved geographic areas.

ENGAGING PARTICIPANTS IN THE GOVERNANCE OF THEIR RESEARCH NETWORKS

PPRNs will have governance structures that rely on patient representation and participation. Affected individuals and their families, along with other stakeholders, will set policies with regard to data use and data sharing within and across the networks, create strategic priorities, and participate in the design and execution of research and its application. While some PPRNs have patients or parents as principal investigators as well as long histories of participant governance, many PPRNs will need to refine or create governance structures and processes that ensure meaningful participant engagement, transparent decision-making, and robust prioritization methods. Attention to engagement principles may be especially relevant in a networked age, with online communities that are more open and diffuse. Engagement with participants may be through 'virtual' or digital media, providing opportunities to reach millions of patients, while challenging existing paradigms based on direct personal contact.

DESIGNING THE DATA INFRASTRUCTURE AND DATA COLLECTION APPROACHES

Enabling participants to collect and use their own data

PPRNs will include mechanisms through which participants can collect and use their own health data. For example, some PPRNs will provide participants with the opportunity to directly enter their information into online surveys within a patient portal, upload data from remote monitoring devices (and sensors), or enter their health data generated by mobile health applications. Some of this patient-generated data may be used for clinical research, and may also help facilitate self-management. Many PPRNs will develop interactive portals that help participants track their exposures and health outcomes over time to gain insights about triggers and recurring symptoms that may further inform conversations with treating physicians.

PPRN members may also be able to share their own electronic health data directly (obtained by providers and hospitals through the View, Download, Transmit (VDT) requirements on Meaningful Use or the Blue Button functions offered by health plans and other data holders). For some PPRNs, however, electronic health record (EHR) data may not be sufficiently granular to capture the clinical condition of interest. Moreover, the ontology for a particular disease may not yet be captured in a standardized format in the medical records. Thus, linking longitudinal data collected by PPRNs from multiple data sources, such as data provided through patient reports, claims data from CMS or private health insurers, clinical registry data, and EHR data is essential,⁵ and finding ways to enable these linkages will be explored and implemented.

Standardizing the collection of patient health data and mapping to the PCORnet common data model

To enable the technical and analytic success of conducting research within PCORnet, a set of data domains will need to be standardized across network partners. In the initial stages, PCORnet will standardize a format for a limited set of demographic data, vital signs, and clinical diagnoses or conditions to a common data model that will allow for multicenter distributed analytics. The PCORnet common data model will not require mapping to a specific clinical terminology, but rather will allow partners to leave the clinical data in the format it was collected (eg, ICD9-CM, ICD9 procedures, HCPCS, SNOMED, LOINC). When harmonization of specific code sets is required (eg, race, smoking status, sex), PCORnet will adopt the relevant national standard for that code set, or select from the several relevant standards based on input from partners and other stakeholders. PCORnet will draw from the experience of the numerous existing distributed networks that use a common data model approach to facilitate complex distributed analytics (eg, HMO Research Network, Vaccine Safety Datalink, and Mini-Sentinel).

The standardization and effective use of patient-generated information, however, provides a formidable challenge, as no standardized ontology or lexicon has been created for the vast majority and range of data elements. PCORnet will provide the infrastructure to create new processes and approaches to the standardization and mapping of these important, yet difficult to incorporate domains, enabling reliable querying across data sources in later phases of development.

ENGAGING PATIENTS IN SHARING THEIR DATA SECURELY WHILE PROTECTING PRIVACY

Attention to security and privacy is critical to develop and sustain patient trust and encourage research participation. The distributed data network approach allows sensitive patient data to remain under the control of the PPRNs (and CDRNs) while enabling analyses to happen behind their secure firewalls. The analyses will typically generate aggregate results that are then shared across networks. Additional security and privacy policies and assurances will be developed by the PCORnet partners. Some PPRNs are exploring a federated informed consent that will be acceptable to multiple institutional review boards. Other PPRNs will be giving each individual participant tools to set granular and dynamic data sharing, privacy and access preferences. Identifying the appropriate balance between data sharing and security/privacy will need to be addressed in the context of existing regulatory requirements, some of which may evolve over time.

ENGAGING PATIENTS TO PRIORITIZE RESEARCH QUESTIONS AND DISSEMINATE RESEARCH RESULTS

PPRNs will be facilitating partnerships between participants and researchers to generate and prioritize research questions as well as optimize the dissemination of research results. This partnership will provide an opportunity to formalize the anecdotal conversations participants have about important health concerns, particularly in conditions where little is known.

One of the unique challenges the PPRNs will face is addressing the different research priorities of PPRN members. While consensus among all members may be difficult, some PPRNs are planning in-person or online discussions of research priorities, including focus groups, and formal methods to rank research topics, which have been shown to be effective in differentiating high from low priority CER topics.⁶

PPRNs will identify promising approaches for disseminating research findings to participants and providers. PPRNs have many mechanisms for disseminating information, ranging from traditional vehicles such as print newsletters and press releases to innovative mechanisms such as social networking and interactive patient portals.

SCALING SMALL NETWORKS INTO A LARGER NETWORK

While traditional patient advocacy organizations have intensively engaged with their communities, maintaining the same level of engagement in more diverse and broad-based communities, such as PCORnet, may be challenging. Small organizations that have built trust as a result of their long-term relationship with the community may have a harder time sharing the more global goals of a larger network. This may be particularly challenging for organizations that represent rare disease communities, which are often self-contained.

These challenges, however, are balanced with the advantages of joining a larger network which offers opportunity for expanding rare disease communities. In addition, many PPRNs share risk factors, co-morbidities, and mechanisms of disease that establish the value and need to integrate the data across PPRN networks within PCORnet. While PCORnet may be able to leverage the trust and commitment of smaller networks, identifying the value proposition for participants and incorporating these values into well-tested outreach strategies and informed consent documents will help sustain the trusting relationship within PCORnet.

IDENTIFYING PATHWAYS TO SUSTAINABILITY

Patient networks that are typically based on participant-centric service models without a robust business model may have difficulty achieving financial sustainability. In PCORnet, PPRNs will generate data to demonstrate their effectiveness and value to participants and clinical researchers, which could lead to viable business models and position PPRNs as competitive recipients of future research grants.

PPRNs will need to address the issue of sustainability in the initial offering to participants to join PCORnet. Pathways to sustainability may include establishing an open-source data platform, creating capacity for longitudinal data collection, integrating EHR datasets, and encouraging ongoing open access to the research community. PCORnet is a national resource that will be open to all funders and investigators for use, and continued organizational support of PCORnet will be critical to enhance and support its patient-centered goals.

CONCLUSION

By the end of the 18-month funding period, PCORnet will be prepared to support multicenter observational and interventional CER studies as well as other types of research at a national scale. PPRNs are providing an unprecedented opportunity for patients, clinicians, and researchers to work together in partnership in all stages of building network capacity. Despite

daunting challenges, the PPRNs are supported by a robust peer network from which to exchange knowledge, build a set of promising practices, and adopt or enhance novel methods. As a result of PPRN efforts, PCORnet will be well-positioned to support meaningful and relevant patient-centered outcomes research that will further inform healthcare decision-making and improve healthcare outcomes.

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