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Patient-Powered Research Networks Aim To Improve Patient Care And Health Research

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ABSTRACT The era of big data, loosely defined as the development and analysis of large or complex data sets, brings new opportunities to empower patients and their families to generate, collect, and use their health information for both clinical and research purposes. In 2013 the Patient-Centered Outcomes Research Institute launched a large national research network, PCORnet, that includes both clinical and patient-powered research networks. This article describes these networks, their potential uses, and the challenges they face. The networks are engaging patients, family members, and caregivers in four key ways: contributing data securely, with privacy protected; including diverse and representative groups of patients in research; prioritizing research questions, participating in research, and disseminating results; and participating in the leadership and governance of patient-powered research networks. If technical, regulatory, and organizational challenges can be overcome, PCORnet will allow research to be conducted more efficiently and cost-effectively and results to be disseminated quickly back to patients, clinicians, and delivery systems to improve patient health.

Big data—loosely defined as the development and analysis of data sets that are large, complex, or both—has become part of health care delivery with the widespread adoption of the electronic health record (EHR), coupled with the explosion of mobile health technologies that are used to generate and manage patient information. The creation of large new data sets has opened the way for the greater use of EHR and mobile health data for clinical and research purposes and for the potential generation of better evidence on treatments for which no, little, or contradictory evidence currently exists.

For patients, their families, and caregivers, the era of big data provides new opportunities to generate and contribute data about themselves and to collaborate with researchers in prioritizing and answering clinical research questions

about the effectiveness of treatments. Patients' participation in research also allows patients to play a significant leadership role in the governance and use of patient-generated data.

More generally, big data contributes to the development of a learning health care system—one that continually incorporates lessons from research into improvements in clinical practice by conducting more pragmatic clinical research studies on a larger scale and more efficiently, in terms of both cost and time.¹

Too much clinical research has been funded, conducted, and published without attention to the ultimate relevance of the research questions or usefulness of the study findings to health care decision makers—namely, patients, their caregivers, clinicians, payers, and policy makers.² Much clinical research has missed the mark, contributing to the common lament that research sits on the shelf instead of being taken

up and translated into practice.²

The Patient-Centered Outcomes Research Institute (PCORI) was authorized as part of the Affordable Care Act to fund comparative clinical effectiveness research.³ The institute's primary goal is to assist patients, clinicians, payers, and policy makers in making better informed decisions about health care and to improve health care delivery and outcomes.

PCORI is an independent nonprofit organization. The twenty-one members of its board of governors are appointed by the Government Accountability Office and represent a broad range of perspectives and expertise in clinical health services research. The board sets PCORI's strategic direction and oversees its activities. The governors have declared that PCORI's research activities will be "guided by patients, caregivers and the broader healthcare community."⁴

The governors decided to fund a national data infrastructure based on EHRs, supplemented with administrative, claims, and patient-generated data. The intention was to build this infrastructure within health care delivery systems to meet PCORI's mission of producing a large volume of comparative effectiveness research.

In July 2012 PCORI convened a national workshop to advance the use of electronic health data.⁵ Invited participants included experts in health informatics and in clinical and health services research, along with federal sponsors of earlier research networks and participants in those networks.^{6,7} Also invited were representatives of patient organizations, particularly those that had formed as online communities for sharing medical information and participating in research.

The notion of an effective and sustainable two-component national research infrastructure was conceived at this workshop. One component was to consist of health system-based networks, while the other was to consist of patient-powered networks. The two components were intended to be distinct and complementary.⁸

The first component includes clinical data research networks that are based on EHR data and other electronic sources related to large patient populations within health care systems, augmented with patient-generated information.⁹ The advantages of creating this component, which combines multiple research groups and patient data, include access to EHR data and the possibilities of hosting clinical trials, engaging clinicians in research, and providing appropriate teams for the dissemination and implementation of research findings.

The second component of the national research infrastructure includes the patient-powered research networks (PPRNs) that originate

in communities of motivated patients with one medical condition or closely related multiple conditions who contribute to the generation of new knowledge for patients with the same condition or conditions.¹⁰ Network members participate in the collection of self-reported and EHR data and in guiding and governing the network's research activities.

By shifting research control from traditional researchers and funders to patients who own their data and can choose to share it, PPRNs have the potential to more systematically address questions of genuine importance to patients. The primary involvement of patients is intended to serve as a creative stimulus to solving longstanding concerns about the US system of regulatory oversight and consent.^{11,12}

Examples of organizations that pioneered the PPRN model include the Dr. Susan Love Research Foundation's Army of Women,¹³ launched in 2008 to engage patients in breast cancer research, and DuchenneConnect, a portal that was started in 2007 by the Parent Project Muscular Dystrophy to connect patients affected by Duchenne or Becker muscular dystrophy—and their families and caregivers—to the medical research community.¹⁴

New Technologies Promote Patient-Generated Data

New technologies are helping empower patients and their families and caregivers to generate large amounts of data about their health, including their experiences and preferences for the management of their condition or conditions. For example, online patient communities use sophisticated web-based and social media platforms for communicating with each other and for gathering, sharing, and analyzing patient-generated information.^{15,16}

Other sources of data include remote monitoring devices such as "wearables"—that is, devices that can be worn on the person. Smartphone apps are also increasingly used to capture many kinds of health information in real time. For example, Fitbit¹⁷ and Jawbone¹⁸ capture data on physical activity and exercise that are then available on smartphone apps. Other devices transmit weight; blood pressure; blood glucose, cholesterol, and oxygen levels; and other physiological information.¹⁹ Increasingly, biospecimens such as saliva, blood, serum, and tissue are being collected and stored by patients (or subcontractors to patient organizations) for research purposes.

Another source of patient-generated data is a result of recent efforts to permit patients to access their own EHRs. Concomitant with the

widespread adoption of EHRs by providers and hospitals,^{20,21} the Office of the National Coordinator for Health Information Technology in the Department of Health and Human Services launched the Blue Button initiative to give patients direct access to and use of their electronic health data—through the ability to view, download, and transmit their coded clinical information to a third party.²²

A number of private health insurers provide administrative claims data to their members who use Blue Button+²³ to allow patients to obtain and share their clinical information for research as well as clinical purposes. Patients' ability to integrate their health information electronically into personal health records is now supported by a number of applications such as Microsoft's Health Vault²⁴ and Cleveland Clinic's MyChart.²⁵

Launching PCORnet

The National Patient-Centered Clinical Research Network, or PCORnet, was created in December 2013, when PCORI funded eleven clinical data research networks and eighteen patient-powered research networks (for details on the twenty-nine individual networks, see the online Appendix).²⁶ Harvard Pilgrim Health Care Institute and Duke University were awarded contracts in September 2013 to establish a Coordinating Center to provide technical and logistical assistance to the twenty-nine network awardees as they build PCORnet. The PCORnet Coordinating Center established eleven task forces linked to a project management office to address challenges related to key areas such as governance, data privacy, and others listed in Figure 1 in the online Appendix.²⁶

The eighteen PPRNs are tasked with engaging patients, caregivers, and families in innovative ways in collecting, using, and governing the use of data. These PPRNs are led by patients who share a medical condition or disorder and who work in partnership with researchers to increase the availability of data on their conditions and to identify appropriate topics for research. The role of patients in these networks varies, with some patients simply contributing data and sharing deidentified data for research purposes, and others involved in the network's leadership and governance.

Major opportunities for PPRNs include learning from each other about how to increase the number of members and retain them, collect data, prioritize research, and work directly with larger health care systems and clinical data research networks. Many PPRNs already provide examples of successful patient engagement in learning networks. For example, a majority of

members of the board of governors of the ImproveCareNow network, which aims to improve health for pediatric patients with inflammatory bowel disease, are parents of patients.²⁷

The PPRNs use a range of different partnership models that link patients and their foundations and associations with researchers who are based primarily in academic research centers. The eighteen networks represent patients with a variety of conditions, including cardiovascular health, mood disorders, and sleep apnea. Of note, about half of the eighteen represent patients with rare diseases. Partnering patient organizations are listed in the Appendix.²⁶ Additional information on each PPRN can be found on PCORnet's website.²⁸

We posit that the success of these networks in partnering with researchers and sponsors to conduct meaningful research will be enhanced when patients and, where appropriate, family members and caregivers are engaged in four key ways: contributing data securely, with privacy protected; including diverse and representative groups of patients in research; prioritizing research questions, participating in research, and disseminating results; and participating in the leadership and governance of the networks.

Contributing Data Securely With Privacy Protections

In theory, the emergence of a multitude of potential sources of health and health care information with big data can provide important data elements for clinical research. Among the important sources are demographic information, diagnoses, prescription data, laboratory results, vital signs, health care utilization, disease severity measures, symptoms and other relevant outcomes, and patients' preferences for treatments and side effects. The transformation and standardization of patient-generated data so that they can be useful for clinical research is fairly new, and it will be some time before investigators can fully leverage the power of these types of data to conduct robust clinical research.

Another potential source of data can be found in existing clinical patient registries, which typically collect clinical details relevant to a specific condition and represent organizational models that PPRNs could adopt and expand upon.²⁹ In fact, a number of PPRNs are exploring strategies to transform existing registries into active patient-led networks in PPRNs.

For example, the pediatric rheumatology PPRN has links to the Childhood Arthritis and Rheumatology Research Alliance Registry and the Pediatric Rheumatology Care and Outcomes Improvement Network, which together have en-

For PCORnet to succeed, it will be critical to communicate transparently with patients and the public about PCORnet's governance.

rolled almost 9,000 patients with pediatric rheumatic disease—a significant proportion of the affected children in the United States. The Crohn's and colitis PPRN maintains an Internet cohort of over 12,500 patients with inflammatory bowel disease—about 1 percent of the people in the United States who have the disease. The Phelan-McDermid syndrome PPRN maintains an international registry that is led by parents and includes 546 participants—49.6 percent of the world's known patients.

For research purposes, it is particularly important to collect patient data longitudinally so that the information generated by delivery systems, health plans, and the patient over time is complete, regardless of changes in the delivery system or health plan coverage. Longitudinality can occur only when disparate sources such as claims data, EHR data, and data provided through patient reports can be linked. But linkage cannot occur unless data are stored in a standardized manner that is common across participating data sources within a network, which allows for the interoperability or exchange of information. Thus, all networks within PCORnet are required to create standardized data that can be understood and queried reliably across data sources and, ultimately, across networks.

Steps to ensure data security and privacy in collecting, storing, and sharing patient data are paramount in building and sustaining patient trust and encouraging patients' participation in research.³⁰ In the planned data-sharing architecture for PCORnet, neither PCORI nor its Coordinating Center will centrally collect or store individual-level, identifiable patient data. Instead, all individual-level patient data will remain under the control of the networks, behind their firewalls. The Coordinating Center will use

distributed analyses to run research queries that will return aggregated (nonidentifiable) data to researchers for analysis.^{31,32} Networks will retain the authority, working with their patient communities, to create pooled individual-level data sets to address particular research questions.

Prior examples of multisite research have helped demonstrate approaches that work for distributed analysis.³³ PCORnet task forces led by the Coordinating Center will develop policies for PCORnet related to security and privacy.

For PCORnet to succeed, it will be critical to communicate transparently with patients and the public about PCORnet's governance. PCORI is setting up processes to ensure that both patient leaders within PCORnet and PCORI's Advisory Panel on Patient Engagement³⁴ will be involved in the review of key PCORnet policies to voice any concerns related to patients' rights, privacy, consent, and autonomy.

Engaging Diverse And Representative Patients In Research

For research purposes, patient data must be available on a sufficiently large population that is representative of the patient population with a given condition. The PPRNs comprise patients who are motivated to participate in research, but they may not be fully representative of all patients who have their condition.²⁸

An important task for the PPRNs is to develop strategies to expand their patient communities and to enhance and report on the diversity and representativeness of these communities. They are being asked to develop effective strategies for reaching out to groups that have historically been underrepresented in terms of race or ethnicity, socioeconomic status, geographic location, health literacy, and clinical severity. These strategies may involve working with health advocacy organizations or health care providers, including the clinical data research networks. The PPRNs will share their best practices associated with patient recruitment and retention through a Patient and Consumer Engagement Task Force led by the Coordinating Center.

Despite some promising practices, there are likely to be many challenges to increasing recruitment, retention, and the representativeness of the membership of the PPRNs. It will be important to explore the incentives for individual patients to become and remain members of the PPRNs. Ensuring that patients feel that the data they are contributing are safe, that privacy and consent issues are adequately addressed, and that patients regard their contributions as meaningful in terms of research they deem important to the management of their conditions are essen-

tial steps in building trust in the process. Strategies for self-management and the increased social support available from fellow patients in these online communities may be another incentive to become PPRN members.

It is clear that there are differences of opinion and levels of comfort across individuals and among the public at large with respect to people's willingness to share personal health information for the purposes of research. For example, a recent study of adult social media users suggests that 92 percent of patients are willing to share data to help researchers learn more about their disease, and that 94 percent believe that their health data should be used to improve the care of future patients who may have the same or a similar condition.³⁵ Nevertheless, 76 percent also raised concerns about the potential use of their data without their knowledge.

Another recent survey, sponsored by the Robert Wood Johnson Foundation, found that among respondents who tracked their health data using wearable devices and smartphone apps, 57 percent required an assurance of privacy for their data to be shared and over 90 percent said that the anonymity of the data was important. Respondents also said that they would be more likely to share their data if they knew that the data would be used only for research for the public good.³⁶

Innovative models of patient engagement that empower patients to decide what levels of information and which data to share, and with whom, are currently gaining traction within patient and research communities.³⁷ These models, in which patients create and manage access to all their information, are promising in terms of future approaches. Patients' willingness to share personal health information is also likely to be higher when the research addresses questions generated and endorsed by the PPRNs.

To succeed, PCORnet must ensure that sufficient protections and transparent policies are in place and well communicated to general public.³⁸ Additional ways of incentivizing patients to join research networks may also emerge as PCORnet develops.

Engaging Patients In Prioritizing, Participating In, And Disseminating Research

The incentives that typically drive the development and conduct of clinical research are generally not aligned with the needs of patients and clinicians.³⁹ The PPRNs are designed as learning networks in which the engagement of patients in all aspects of research should increase the usefulness of the research findings and their imple-

mentation into health care practice, to contribute to improving patients' health outcomes.⁴⁰

Eliciting research questions directly from patients and their caregivers and clinicians and asking them specifically what matters to them in their daily lives has the potential to focus clinical research activity on more practical, patient-centered questions and outcomes, compared to traditional, investigator-generated research questions.⁴¹ The PPRNs are expected to elicit research questions from their members.⁴² It will be a challenge to ensure that the prioritization process for research questions is accessible to people with a wide range of educational backgrounds and expertise.

Speed and rate of recruitment of patients for clinical trials has long been a challenge for clinical research.⁴³ There have been successful models in engaging patients more actively in research, such as the Army of Women,¹³ which aims to recruit men and women who are willing to participate in breast cancer research. As of May 2014 this organization had enrolled 376,001 participants who were willing to be contacted for research studies,¹³ and between July 2012 and December 2013, researchers conducting at least thirty-five studies worked with Army of Women participants to reach their recruitment targets.⁴⁴

Improving rates of patient participation in research will require informing prospective participants clearly and transparently about the risks and benefits of research participation and obtaining permissions in a manner that protects human rights. Challenges remain in the ethical and oversight system for patient participation in health research, a system that has been described as cumbersome and expensive.⁴⁵

Several PCORnet task forces will facilitate sharing best practices and explore the use and acceptability of central Institutional Review Boards (IRBs) or Internet-facilitated shared review systems and centralized support for enrolling subjects and obtaining their consent.⁴⁶ For example, some PPRNs will, through a website, allow each participant to set very specific as well as tailored preferences for data sharing, privacy, and access.⁴⁷ In these cases, each participant will specify who may access and make use of their identities and for what purpose, and who may export data from the registry to a third-party platform. During 2014 the Coordinating Center's Ethics and Regulatory Task Force will provide specific guidance regarding models for IRB oversight and systemwide human research protection approaches for PCORnet.

The era of big data is providing new opportunities for patients and their families to be active participants in research.

Governance Of Patient Research Networks

Meaningful patient involvement in network governance is critical to the success of the PCORnet research networks. Each PPRN is charged with developing a governance structure that includes patients in leadership positions and operating policies that ensure that patient control such tasks as choosing research topics, signing data use agreements, and making decisions about the network's participation in proposed studies.

The eighteen PPRNs have created a variety of governance models. For example, the Phelan-McDermid syndrome PPRN is led by patients and has a parent advisory committee that is charged with ensuring that its registry—which collects clinical data on their patients—meets the needs of families and is in alignment with the community's goals. The Community-Engaged Network for All PPRN uses participant-led governance models that bring leaders and people affected by each medical condition together to oversee their network.⁴⁸

Each PPRN faces different challenges in finding effective ways to involve patients in governing the network and controlling the uses of their data, since the PPRNs are at different points in the process of setting up their organization. Indeed, the networks will need to choose governance models that respect their current structure, history, and culture while balancing the requirements of participating in a large-scale national network such as PCORnet.

PCORnet engages patients in its own governance by having patient representatives serve on both its Steering Committee and its Executive Steering Committee. A PCORnet Patient "Tiger Team" (a small team that focuses on a specific set of critical issues) composed of PCORI staff, the

patient representatives from the Coordinating Center, the Steering Committee, and the Patient Engagement Task Force leaders will address issues relating to patient engagement and participation that arise in PCORnet itself, as distinct from individual member networks. The involvement of patient leaders within PCORnet and PCORI's Advisory Panel on Patient Engagement (independent from PCORnet) is an important way to scrutinize key PCORnet policies as they develop to ensure that the voice and perspective of patients are front and center of PCORnet as an organization and to help build public trust in the enterprise.

Challenges And Limitations

The PCORnet networks held their kickoff meeting in January 2014. The eighteen PPRNs will need to address many challenges. These include collecting relevant clinical data, including information from patient self-reports and from their network's EHRs; harmonizing data so that they can be useful to PCORnet as a whole; increasing the numbers of patients with a single condition who are registered and willing to share data, participate in research (including interventional studies), prioritize research questions, and disseminate results; establishing patients' trust with respect to the privacy and security of their personal information; streamlining the consent and IRB processes while protecting patients' rights; and meaningfully engaging patients in the networks' governance.

For PCORnet as a whole, the challenges include successfully supporting the diversity, approaches, expertise, and culture of each individual network while ensuring that a national and functional network is built. A significant challenge will be to explore mechanisms for the sustainability of PCORnet and its individual networks. PCORI has made a \$102 million investment in the initial investment phase (March 2014–September 2015). Some maintenance funds will likely be available from PCORI in a second phase of funding for networks that have been successful in the first phase. However, PCORI will not continue to fund the infrastructure of PCORnet at the same levels in later phases.

The sustainability of PCORnet will eventually depend on the willingness of a variety of research funders to use it to fund research. For the clinical data research networks, the engagement of health system leaders in supporting them will be critical. For the PPRNs, we expect to see a variety of models of network sustainability, reflecting the diversity of the member organizations. The issue of sustainability for the individ-

ual networks and for PCORnet as a whole is a real concern.

PCORnet's Metrics Of Success And Evaluation Plan

PCORnet's overall goal is to build a network across the United States that will support the rapid, efficient, and cost-effective conduct of research. A metric of success will be the willingness of external funders, both public (for example, the National Institutes of Health) and private (such as the pharmaceutical and device industries), to fund research studies using PCORnet. The funders' willingness will depend on whether PCORnet achieves true gains in the volume, completeness, accuracy, and access to standardized and interoperable demographic and clinical data and in the ability to recruit patients to participate as subjects in interventional research.

The first phase of PCORnet extends through September 2015. PCORI has contracted with investigators at the RAND Corporation to conduct an independent evaluation of PCORnet to provide an objective summary of its capabilities at the end of the initial eighteen-month period and a qualitative analysis of the attributes that supported or hindered its progress. The domains to

be assessed in the evaluation include individual network leaders' participation in developing and implementing PCORnet policies and practices, the alignment of clinical and patient network activities with PCORnet priorities, and the readiness of PCORnet to conduct clinical trials as well as observational research studies. This assessment of PCORnet will provide detailed information with which to better understand its progress by September 2015.

Conclusion

The era of big data is providing new opportunities for patients and their families to be active participants in research. If technical, regulatory, and organizational challenges can be overcome, PCORnet will enable the conduct of research that is more relevant to patients and more efficient, thereby fulfilling the promise of the learning health care system.

Sharing study findings with patients on questions they prioritized and for which they contributed data will be paramount. Innovative ways to share sometimes complex information from big data with patients, families, and other nontechnical stakeholders will need to be further explored. ■

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