

National Patient-Centered Clinical Research Network (PCORnet) Phase I

Final Evaluation Report

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Preface

The Patient-Centered Outcomes Research Institute (PCORI) is an independent, nonprofit, nongovernmental organization authorized under the Affordable Care Act of 2010 and funded by Congress to help close the gaps in research evidence needed to improve key health outcomes. To do this, PCORI identifies critical research questions, funds patient-centered clinical comparative effectiveness research (CER),¹ and strives to disseminate the results in ways that providers and patients will find useful. In recognition that the nation's capacity to conduct CER rapidly and efficiently remains extremely limited, and to significantly increase the amount of information and the speed at which it is generated, PCORI invested more than \$100 million in the development of PCORnet, the National Patient-Centered Clinical Research Network. When fully established, PCORnet will be a large, highly representative national network for conducting clinical outcomes research.

The first 18 months of PCORnet's development (March 2014 through August 2015) was dedicated to building its governance, technical, and research infrastructure. As part of this developmental phase, PCORI desired an external independent evaluation to help it assess PCORnet's progress. This report describes the findings of this formative evaluation of PCORnet's Phase I activities and addresses PCORnet's readiness to achieve PCORI priority objectives in Phase II (which began in September 2015). The evaluation was conducted under contract to PCORI and with its input—but the conclusions drawn are those of RAND investigators alone and do not necessarily represent the views of the institute's Board of Governors, executive director, or staff.

The findings should be helpful in illuminating lessons learned from PCORnet's implementation, the current state of readiness of the network, and priority areas for attention (and any necessary remediation) in the earliest stages of Phase II.

This report should be of particular interest to the PCORI Board of Governors, executive director, and staff; PCORnet participants; federal policymakers, such as Congress; and the agencies of the Department of Health and Human Services, such as the National Institutes of Health and the Food and Drug Administration (who have been collaborating with PCORnet since its inception). It should also be of interest to funders of CER, the pharmaceutical and device industries, health system leaders, clinicians, and health services researchers, who have been invited to help guide PCORnet's formation and may benefit from future collaborations with PCORnet.

¹ Zika et al., 2011.

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Summary

Background

The Patient-Centered Outcomes Research Institute (PCORI) is an independent, nonprofit, nongovernmental organization authorized under the Affordable Care Act of 2010 to help close gaps in research evidence that are needed to optimize patient and clinician decisionmaking and improve health outcomes. To do this, PCORI identifies critical research questions, funds patient-centered clinical comparative effectiveness research (CER), and strives to disseminate the results in ways that providers and patients will find useful. In recognition that the nation’s capacity to conduct CER rapidly and efficiently remains extremely limited, PCORI invested more than \$100 million to establish PCORnet, the National Patient-Centered Clinical Research Network.

PCORnet represents a new research paradigm. PCORnet is designed to utilize a combination of resources and strategies to create a multiplier effect in health and health system research. By leveraging relationships and partnerships between organizations, developing a data infrastructure to support data-sharing, cultivating a patient-centered research focus, and enhancing the visibility and utility of CER, PCORnet aims “to respond to [patient] priorities and speed the creation of new knowledge to guide treatment on a national scale.”² Accessing vast amounts of clinically rich data from electronic health records (EHRs) will accelerate, lower the cost, and improve the patient-centeredness of clinical research. By drawing on data for tens of millions of patients, the network has the potential to strengthen research on rare diseases and expand research on subpopulations that have limited representation in current health outcomes research. Moreover, by integrating clinical research with the routine delivery of care, PCORnet seeks to speed the implementation of CER study results—a key limitation of the current research paradigm that delays patient access to beneficial treatments.

The centerpiece of the PCORnet initiative is a distributed research network that combines clinical data from EHRs and data contributed directly by patients from participating networks located throughout the United States. A distributed network allows a spectrum of analyses to be conducted without the physical pooling of data, which remain behind the protection of each institution’s firewalls and covered by an individual network’s own data safety, security, and governance policies. Unlike a centralized database, a distributed network requires individual networks to design data files in a standard format (called the common data model) that allows network partners to query data from across the entire network. The overall vision is that collaborative agreements will eventually allow even those researchers not directly affiliated with PCORnet to use its distributed research network to conduct research on treatments for a wide

² PCORnet, undated(o).

range of clinical conditions and health system delivery interventions concurrently with the broad participation of patients, clinicians, and health systems.

Evaluating PCORnet

The first phase of PCORnet's development (March 2014 through August 2015) was dedicated to building the governance and technical infrastructure necessary to support the observational research studies and clinical trials in its operational phase. As part of this 18-month developmental phase, PCORI required an independent external evaluation to assess implementation of the network, including progress on developing an effective network governance infrastructure, implementing the distributed data network, streamlining research oversight processes, promoting cross-network collaboration, engaging stakeholders, and other related activities. PCORI contracted with the RAND Corporation to conduct a formative evaluation of PCORnet's Phase I activities. The evaluation's goals were threefold: (1) to identify the key accomplishments and challenges in implementing PCORnet within key activity areas, (2) to gather information on the experience of PCORnet participants and stakeholders and their perceptions of implementation progress during Phase I, and (3) to synthesize available information to assess the extent to which PCORnet was on the path to research readiness by the end of Phase I. These findings would be useful to PCORI in determining whether PCORnet would be well positioned to achieve its priority objectives in Phase II.

RAND developed an evaluation framework comprising three domains: The *implementation approach* examines whether the structures were in place to guide implementation activities and allows an assessment of whether implementation of specific components of PCORnet occurred as intended. The *implementation process* focuses on the implementation challenges that may have arisen in the course of executing PCORnet's implementation plans and includes both barriers and facilitators in overcoming these challenges from the perspective of participants and stakeholder groups. The *implementation outcome* includes an assessment of the extent to which the implementation plan was successfully executed and the Phase I goals were achieved.

Because quantitative data on various aspects of PCORnet's implementation are limited at this juncture, the evaluation relies primarily on observations of PCORnet's Steering Committee, Executive Committee, and task force meetings and communications; key informant interviews with over 170 unique individuals who have been participating in PCORnet activities; and the review of quarterly progress reports submitted to PCORI by the networks participating in PCORnet. Some quantitative data describing the development of PCORnet's data infrastructure as of the end of Phase I were provided by PCORI and the PCORnet Coordinating Center (described below). The RAND research team synthesized the information gathered from observations, interviews, document review, and the quantitative data provided to the evaluation team to develop a cohesive picture of the current status of the network, with an emphasis on the readiness for PCORnet to initiate research by the end of Phase I.

The external evaluation covered approximately the first 16 months of the Phase I period and represents a point-in-time assessment of a network that was undergoing constant evolution and change—particularly toward the end of Phase I. As a result, some of the challenges identified in this report will already have been addressed by the time this report is published. However, we share these challenges in this report because they represent opportunities for learning by current PCORnet participants, future PCORnet participants, and others who might seek to undertake an initiative similar to PCORnet. Other readiness challenges reported here represent opportunities for PCORnet course corrections, remediation, or future enhancements in Phase II.

PCORnet’s Participants

Before describing the development of PCORnet’s operational capacity, we briefly describe the participants in PCORnet. In December 2013, PCORI’s Board of Governors authorized funding for 11 Clinical Data Research Networks (CDRNs) and 18 Patient-Powered Research Networks (PPRNs) to become the initial networks comprising PCORnet. A CDRN is a collaboration of health systems tasked with identifying patient cohorts and creating, maintaining, and standardizing electronic resources to support PCORnet research activities. The 11 CDRNs participating in Phase I include delivery systems that are located in diverse regions of the United States and cover populations ranging from 1 million to 28 million patients each.

A PPRN is composed of patients, caregivers, and families who are “motivated to build an ideal network [of stakeholders] and play an active role in patient-centered comparative effectiveness research.”³ Most of the 18 PPRNs participating in Phase I consisted of organizations that were motivated by a shared medical condition and highly engaged patient communities to form a network designed to advance the state of science by incorporating stakeholder voices into the research process.

In addition, PCORI funded a Coordinating Center to oversee critical elements of PCORnet’s development, provide technical assistance, and facilitate the establishment of the network during its first 18 months. Additional CDRNs and PPRNs were funded at the end of Phase I and have joined PCORnet for Phase II.

Progress Developing Research Readiness

The balance of the report focuses on the development of PCORnet’s operational capacity, beginning with the development of a functional governance structure and the data and research infrastructures necessary to conduct multi-site observational research and clinical trials. We then discuss the extent and nature of collaborations within the network during Phase I, as well as efforts to engage a broad set of stakeholders in the design, use, and sustainability of the network.

³ PCORI, 2013a.

Below we summarize the results of RAND’s evaluation of PCORnet’s Phase I activities in each of these five areas.

PCORnet’s Governance Infrastructure

Governance is key to the ability of any network to function. It includes the norms and rules for interacting, making decisions, resolving conflicts, and developing the policies and procedures that are necessary to achieve any sort of collective action. Without agreement on a governing structure (and how it will function), progress toward other goals (especially on a tight time frame) can easily be stymied.

One of PCORnet’s first tasks was to quickly establish a governance structure for a large and complex research enterprise that had little precedent. The structure developed to govern this network of 29 self-organized smaller networks consisted of a representative Steering Committee, its Executive Committee, and an advisory Patient Council. The Executive Committee was designated by the Steering Committee to develop strategies and processes for PCORnet implementation. Under contract to PCORI but taking direction from the Steering Committee was a Coordinating Center providing technical and logistical support. Eleven task forces established by the Coordinating Center, and populated by CDRNs and PPRNs, were designed to assist in policy development and sharing of implementation strategies between networks around such topics as data privacy and biobanking. The Steering Committee was intended to “guide members of PCORnet and advise PCORI leadership” but would be “subject to the oversight of PCORI.”⁴

The development by PCORI, the Coordinating Center, and the 29 networks of a governance structure to guide the many infrastructure-building activities described in this report is a substantial achievement in its own right. However, PCORnet participants reported confusion about the initial governance of the network—about who held the “real” decisionmaking authority and about their ability to contribute to the important decisions—and described a perceived lack of transparency. Most interview participants (including PCORnet and Coordinating Center leadership) expressed the view that PCORnet needed a revised governance structure for Phase II that could “efficiently and effectively make decisions for the entire network.”

Over the course of Phase I, PCORnet’s governance has demonstrated an ongoing ability to course-correct after recognizing that certain structures or processes were either inefficient or ineffective. The most recent and obvious example was the decision to empower PCORnet’s Steering Committee (and its Executive Committee arm) with greater decisionmaking authority, which helped to allay concerns that the network’s governance structure was not meeting the needs of participants while also laying the groundwork for a future model of self-governance. In addition, by reconfiguring the Coordinating Center’s task forces as work groups under the oversight of the Executive Committee, PCORI took steps to address ongoing governance and

⁴ Fleurence, Curtis, et al., 2014b.

operational challenges more effectively through smaller groups that have greater accountability and oversight. Finally, PCORI's decision to assume leadership for the development of draft policies as part of an Executive Committee work group was an attempt to better align policies with the evolving governance structure and to simplify a cumbersome process. All of these changes gave PCORnet a perceptible boost in momentum and gave those participants involved in the governance operations the expectation that future operational challenges could be resolved more quickly.

PCORnet's new governance model was approved on the last day of Phase I by the Steering Committee, and its implementation and effectiveness should be examined closely in Phase II. There is great enthusiasm for the new model but also some concern that without strong executive leadership that instills a strong PCORnet identity, individual networks will be less inclined toward PCORnet level goals and will instead focus on their own network's needs, thereby undermining the viability of the joint enterprise.

PCORnet's Data Infrastructure

Standardizing Data

All CDRNs and PPRNs were expected to extract patient data from various sources, transform each data element in accordance with the PCORnet common data model, and load the transformed data into one or more local "DataMarts" (databases) that are maintained securely behind each organization's firewalls to support cross-network querying. Despite initial frustration with the selection of the common data model and the significant resources required to map native formats to the common data model, most networks reported few technical challenges standardizing their data.

Both CDRNs and PPRNs made considerable progress establishing DataMarts, with 89 percent of CDRN DataMarts and 68 percent of PPRN DataMarts set up by the end of Phase I. CDRNs were able to standardize data for a total of 72.6 million patients, representing over 3 billion unique patient encounters. In addition, all CDRNs met their Phase I requirement of standardizing data for over 1 million patients. Comparable data for PPRNs were not available, but PPRNs reported standardizing fewer domains of the common data model because of challenges PPRNs faced in obtaining EHR data from their patients and because PPRNs were required to standardize fewer domains of the common data model.

During Phase I, PCORnet developed a transparent and participatory process to guide expansions to the common data model—a process that was used twice during Phase I. However, the limited number of data elements in the common data model is a concern to both CDRNs and PPRNs and may limit the type of research that PCORnet can undertake until the common data model is enriched with additional data elements.

Developing PCORnet's Querying Capability

Nearly 75 percent of CDRNs and 26 percent of PPRNs had set up both the infrastructure and processes necessary to field, execute, and return data queries from the Coordinating Center by the end of Phase I. The low rate of progress among PPRNs may reflect the fact that PPRNs varied in their baseline data capabilities when compared with CDRNs and their slower progress in setting up DataMarts. Continuing to establish and test PCORnet's querying capabilities should remain a high priority for the early Phase II period.

Ensuring Data Quality

To ensure that PCORnet data could support high-quality research, CDRNs and PPRNs developed systematic approaches to assess and improve data quality. Duplicate records (caused by patients seeking care from multiple organizations within a CDRN) appear to be the biggest data-quality challenge facing some networks, and a group of networks is actively pursuing strategies to address the issue.

The Coordinating Center's strategy of assessing data quality was to use "data characterization" queries to assess compatibility with the common data model and to conduct numerical and logical checks of the data. However, only two DataMarts had undergone the testing process by the end of Phase I. The slow progress in assessing data quality appears to be due to the need for one-on-one engagement between the Coordinating Center and each network to review data quality issues and the need to test and refine the querying process before bringing it to scale. As a result, the quality of PCORnet's data could not be summarized quantitatively. Efforts to assess data quality should continue to receive high priority in Phase II.

Ensuring Data Completeness

CDRNs were expected to have complete data on their million-member populations by the end of Phase I—primarily by obtaining claims data covering their population. Challenges to improving the completeness of the data in their DataMarts included the restrictiveness of existing data use agreements, the cost of acquiring and cleaning claims data, and the lack of a compelling value proposition for payers to collaborate on data linkages. During Phase I, CDRNs actively engaged commercial payers, the Centers for Medicare & Medicaid Services (CMS) and state Medicaid agencies to pursue linkages to administrative and claims data to address gaps in their EHR data. These efforts have yet to increase the volume of claims data within PCORnet. However, an Executive Committee work group has developed a potentially useful framework that addresses specific governance and technical challenges that may lead to more extensive collaborations with payers during Phase II. Efforts to accelerate engagement with payers may be warranted, given the importance of complete data for nearly all types of PCORnet research.

Developing Data Privacy Standards

CDRNs and PPRNs were required to develop privacy protections governing data held within their networks. While most networks have made progress developing general policies in this area, some have postponed further policy development because of the lack of guidance from the Data Privacy Task Force, which stopped meeting midway through Phase I. Some networks reported challenges developing strategies to minimize the risk of reidentifying patients when responding to queries or certifying that their deidentification strategies were sufficiently rigorous. Identifying solutions to these challenges and continued work developing PCORnet-level data privacy policies, such as minimum standards for all networks, should be a priority for Phase II.

Collecting Patient-Generated Data

All networks were required to collect a core set of patient-reported outcomes (PROs) (the PCORnet Common Measures) and could supplement their local data resources with other patient-generated data (e.g., biomarkers, health status, health behaviors) to support future research. While quantitative summaries of the extent to which PRO data were collected and integrated within CDRN and PPRN DataMarts were not available as of the writing of this report, PPRNs reported being actively engaged in the collection of (or have developed plans to collect) substantial amounts of patient-generated data. PPRNs typically are using multiple instruments and multiple modalities to support collection of PROs. To address the concerns of multiple participants who viewed the PCORnet Common Measures as having limited utility because they represent individual items selected from multi-item scales, future updates to the common data model might include entire PRO measurement scales.

Developing Biobanking Infrastructure

Neither CDRNs nor PPRNs were required to develop biobanking capabilities during Phase I, as other infrastructure building activities assumed greater priority. Nevertheless, many CDRNs and PPRNs developed committees and initiated other planning activities to support the development of these capabilities in Phase II; CDRNs and PPRNs that already had biobanking operations continued their collection of biospecimens. Creating an inventory of biospecimen data within PCORnet and establishing standard operating procedures (SOPs) to facilitate the use of biospecimens in PCORnet research should receive higher priority for Phase II to expand the types of research that PCORnet is able to support.

PCORnet's Research Infrastructure

Implementing Multi-Site Institutional Review Board Review Processes

CDRNs were expected to develop streamlined institutional review board (IRB) review processes using any approach that was considered appropriate for their local network. While some CDRNs had delays or difficulty obtaining buy-in from all sites within their network, all CDRNs made progress implementing multi-site IRB models during Phase I. Many CDRNs were already pioneers in developing these models prior to joining PCORnet, which helped to speed implementation. Some CDRNs reported that cultural changes might be under way in their individual institutions, as IRB leaders began questioning their existing review processes over the course of Phase I. These review processes will face their first true test during Phase II, when PCORnet begins conducting interventional studies on a larger scale. PCORnet's decision near the end of Phase I to recommend a common multi-site IRB review model across the network in the pursuit of greater efficiency may also pose challenges among CDRNs that adopted alternative models during Phase I.

Implementing Patient-Centered Consent Processes

CDRNs and PPRNs did not face specific requirements to enhance informed consent processes, but the broad participation of patients in the network provided a unique opportunity for innovation in this area. Some CDRNs developed consent templates for their networks, while a few other CDRNs developed innovative tablet-based applications (apps) to enhance their consent process using videos and other media and instituting tests of comprehension at the culmination of the consent process. PPRNs have made even greater progress developing electronic consent processes, many of which feature tools that allow patients to consent to specific forms of data collection and uses of their data. Many networks have laid at least some groundwork for making progress during Phase II, although institutional differences in consent processes and in the interpretation of federal regulations suggest that additional engagement around this issue across networks could be useful in Phase II.

Enrolling Patients into Cohorts

CDRNs were expected to enroll patients into an obesity cohort and both a rare-disease and common-condition cohort of their choosing, while PPRNs were required to meet specific patient enrollment targets by the end of Phase I. Enrolling patients into these cohorts would jump-start research projects by having patients "ready" to participate in research (as documented through responses to surveys that PPRNs and CDRNs were required to conduct during Phase I).

Strategies for recruiting patients varied across CDRNs and PPRNs and included both in-person recruitment in clinic settings and electronic recruitment. PPRNs used a broader set of strategies, including social media and Internet-based methods, and reported a variety of ways to

keep patients engaged in network activities, including novel uses of their patient portals. CDRNs and PPRNs faced a variety of recruitment challenges, including problems effectively communicating the purpose of PCORnet, competition for patients with other research initiatives, and difficulty recruiting diverse patients. It is unclear at this point what proportion of these patients are willing to participate in PCORnet research because of slow progress in completing the required cohort surveys.

Creating a Collaborative Culture

Cross-network collaboration during Phase I was considered vital to the development of a culture within PCORnet that embraced cross-network research while also allowing individual networks to pursue research priorities locally. CDRNs reported collaborating with an average of 11 networks, and PPRNs reported collaborating with an average of 7 networks. Collaborations spanned patient identification, referral, and recruitment activities; data standardization and linkage; research studies; and broad-based collaborations around multiple topics.

CDRNs and PPRNs experienced numerous challenges engaging in these collaborations. Both types of networks noted that the articulation of specific roles that CDRNs and PPRNs could play in collaborations was missing for most of Phase I and could have helped to guide collaborations early on. Time pressure to complete each network's Phase I milestones, tight Phase I budgets that prioritized infrastructure-building activities over collaboration, differences in networks' readiness to collaborate, and the lack of a clear value proposition for CDRNs to work with PPRNs so that both parties would find collaborations to be "mutually beneficial" were a deterrent for some networks. In addition, the lack of an aggressive approach to promote collaborations and the lack of affinity groups or tools to support collaborations may have undermined these efforts.

While most participants believe that the majority of CDRNs and PPRNs have embraced PCORI's vision that the network should function primarily as a national resource for CER, some participants remain concerned that the level of collaboration activity—particularly between CDRNs and PPRNs—may be suboptimal. However, CDRNs are obligated to collaborate with PPRNs as a condition of their Phase II funding, and the appointment of an additional principal investigator to the Coordinating Center to coordinate PPRN activities may ultimately strengthen the role of PPRNs in the network as a whole. A mature data infrastructure in which both CDRNs and PPRNs can leverage their complementary strengths may be the most important facilitator of greater collaboration between CDRNs and PPRNs in Phase II.

Engaging Stakeholders

Engaging Patients

Patient representatives served in both PCORnet-level and local governance roles during Phase I. Patients who served on PCORnet-level governance bodies (primarily Coordinating Center task forces) reported mixed experiences. Some reported that their voices were being heard, while others reported that their lack of experience or knowledge prevented them from engaging at the same level as other stakeholders. CDRNs and PPRNs provided assistance to help patients participate in these activities. Some stakeholders viewed patients' participation in decisionmaking as notable, whereas others questioned the degree to which patients were truly integrated and, as a result, may not have made many substantive contributions. Some patient representatives served on PCORnet's Patient Council and reviewed draft policies to ensure that they protected the interests of patients; however, members of the council had to aggressively engage PCORnet's other governance entities to ensure that their feedback was incorporated.

Patient representatives also served in a variety of local governance roles during Phase I. Patients developed and reviewed content destined for websites, patient portals, and surveys; prioritized topics for studies; and contributed to some aspects of study design. PPRNs allocated a much larger share of governance responsibilities to patients, which was designed to allow patients and nonpatients to have equal weight in local decisionmaking. PPRN patient representatives were also more likely to report making specific contributions to their PPRN. Several patient representatives indicated that formal training would be helpful in the future to help patients fulfill their governance roles.

Engaging Clinicians

The ability of PCORnet to conduct clinical trials on a large scale requires the active participation of clinicians—particularly within each CDRN. The responsibility for engaging clinicians fell almost exclusively to CDRNs and PPRNs, who used a range of strategies to do so, including providing information, tools, and resources to clinicians in return for their participation; discussing research priorities; and working to align research and clinical workflows. While it is difficult to assess the intensity or outcome of clinician engagement efforts during Phase I, engagement of clinicians appears to have been less robust than that of other stakeholder groups. Clinicians' busy schedules, the limited role that clinicians played in many networks' patient recruitment plans, and perceptions by clinicians that PCORnet represented “competition” for scarce research dollars were noted as barriers to engagement. The completion of demonstration projects will be critical to demonstrating the value of the network to clinicians.

Engaging Health System Leaders

The health systems partnering within each CDRN are vital to the success of PCORnet because they provide both EHR data and the infrastructure for clinical trials, they represent a key source of sustainability funding, and they are integral to future dissemination efforts. While directly eliciting the perspectives of health system leaders was beyond the scope of the evaluation, interviews with CDRN principal investigators indicated that CDRNs have worked to articulate PCORnet's value proposition for health systems and conducted outreach to health system leaders during Phase I. Overall, PCORnet has also made progress by laying the groundwork for closer engagement between CDRNs and health system leaders during Phase II by convening an Institute of Medicine (IOM) meeting of leading health system executives and mobilizing additional funding to support greater engagement with health system leaders. The stated intent is that these efforts will culminate in PCORnet demonstration projects on topics that are considered high priority by health system leaders.

Engaging Federal Stakeholders

Representatives of several federal agencies served in governance roles (primarily as Steering Committee members) during Phase I to ensure that PCORnet's development takes into account the needs of future funders of PCORnet studies or users of its data. Most federal partners reported being satisfied with their governance roles during Phase I, but they identified several governance and data challenges for PCORnet to address as it moved into Phase II—particularly the network's ability to make decisions that appropriately balance stakeholder priorities efficiently. The limited breadth of PCORnet's current data resources also raised some concerns, and federal partners expressed hope that PCORnet would expand its data model, accommodate diverse funding streams, and develop better estimates of the cost of future research and general operations to support sustainability planning. Despite these concerns, most federal partners shared the opinion that PCORnet was generally “on the right track.” Others noted that it may be too soon to tell because PCORnet's first few demonstration projects, which were considered key to demonstrating the network's capabilities, are still in their early stages, and PCORnet's governance structure continues to evolve.

Engaging Industry Stakeholders

PCORI also engaged representatives of the pharmaceutical, device, and health insurance industries to guide PCORnet's implementation. As of the end of Phase I, several industry stakeholders remained uncertain as to how PCORnet research studies would be initiated and conducted and preferred to take a “wait and see” approach to the network before drawing conclusions on the network's potential value to their industries. As a result, continuing to communicate the value proposition of partnership and quickly establishing PCORnet's capabilities through current and future demonstration projects should be high priorities for the

network in Phase II. Several initiatives launched near the end of Phase I suggest that PCORnet has laid the groundwork for closer engagement. These include appropriation of funding for demonstration projects that will engage health plans specifically; the creation of a work group that will develop SOPs for collecting, evaluating, and triaging research opportunities; and the creation of a work group that is focused specifically on promoting collaboration with industry partners that will culminate in one or more demonstration projects.

Conclusion

In the face of high expectations, PCORnet made substantial progress during its initial phase of development and operations. Many of the critical structural pieces of the network were implemented. However, many challenges remain. The degree to which PCORnet can scale up quickly in Phase II by expanding the common data model and launching studies that are supported by high-quality data and present compelling use cases to potential funders will determine the network's future success. As PCORnet's infrastructure-building phase transitions into a new phase characterized by high levels of research activity, its evaluation needs will change significantly. Opportunities exist for a robust and rigorous evaluation in Phase II—including the collection of quantitative metrics of performance—which both can help PCORnet meaningfully track its progress over time and can also be used to support future external evaluation efforts.

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Abbreviations

ACA	Affordable Care Act
ACP	Accelerated Cure Project for Multiple Sclerosis
AdvaMed	Advanced Medical Technology Association
AHIP	America's Health Insurance Plans
AHRQ	Agency for Healthcare Research & Quality
ASAA	American Sleep Apnea Association
ASPE	Assistant Secretary for Planning and Evaluation
AWAKE	Alert, Well, And Keeping Energetic
BCSC	Breast Cancer Surveillance Consortium
BOLD	Back pain Outcomes using Longitudinal Data
C3N	Collaborative Chronic Care Network
CAHPS	Consumer Assessment of Healthcare Providers and Systems
CCFA	Crohns and Colitis Foundation of America
CDC	Centers for Disease Control and Prevention
CDRN	Clinical Data Research Network
CER	comparative effectiveness research
CMIO	chief medical information officer
CMO	chief medical officer
CMS	Centers for Medicare & Medicaid Services
CORRONA	Consortium of Rheumatology Researchers of North America
CRN	Cancer Research Network
CTSA	Clinical and Translational Science Awards
DRN	distributed research network
DSSNI	Data Standards, Security, and Network Infrastructure Task Force
DUA	data use agreement
ED	emergency department
EHR	electronic health record
EMR	electronic medical record

ETL	extract, transform, and load
FDA	U.S. Food and Drug Administration
GHLF	Global Health Living Foundation
HHS	Department of Health and Human Services
HIE	health information exchange
HIPAA	Health Insurance Portability and Accountability Act
HMO	health maintenance organization
HMORN	HMO Research Network
HMORN-VDW	HMO Research Network Virtual Data Warehouse
HSIS	Health Systems Interactions and Sustainability Task Force
i2b2	Informatics for Integrating Biology and the Bedside
IBD	irritable bowel disease
IBQ	initial basic query
IOM	Institute of Medicine
IRB	Institutional Review Board
MDMA	Medical Device Manufacturers Association
MRA	Master Reliance Agreement
NCI	National Cancer Institute
NIH	National Institutes of Health
NPC	National Pharmaceutical Council
OHRP	Office of Human Research Protections
OMOP	Observational Medical Outcomes Partnership
ONC	Office of the National Coordinator for Health Information Technology
PCE	Patient and Consumer Engagement Task Force
PCOR	patient-centered outcomes research
PCORI	Patient-Centered Outcomes Research Institute
PCORnet	National Patient-Centered Clinical Research Network
PEDSnet	National Pediatric Learning System
PHI	protected health information
PhRMA	Pharmaceutical Research and Manufacturers of America
PI	principal investigator
PMO	Project Management Office

PMSF	Phelan-McDermid Syndrome Foundation
PPRN	Patient-Powered Research Network
PRIMIER	Patients Receiving Integrative Medicine Interventions Effectiveness Registry
PRO	patient-reported outcome
PROMIS	Patient Reported Outcomes Measurement Information Systems
RA	rheumatoid arthritis
RFA	request for application
SAS	statistical analysis software
SCILHS	Scalable Collaborative Infrastructure for a Learning Health System
SOP	standard operating procedure
SQL	structured language query
UC ReX	University of California Research eXchange
UDF	universal disk format
VDW	virtual data warehouse

1. Introduction

Authorized by Section 6301 of the Patient Protection and Affordable Care Act (ACA), the Patient-Centered Outcomes Research Institute (PCORI) is intended to “assist patients, clinicians, purchasers, and policy-makers in making informed health decisions by advancing the quality and relevance of evidence.”⁵ A key mechanism by which PCORI has sought to achieve this purpose and advance the field of health research is through the development of PCORnet, the National Patient-Centered Clinical Research Network. Described as a “network of networks,” PCORnet is designed to facilitate the close collaboration of health systems, clinicians, patients, and other stakeholders to guide, perform, and disseminate research that matters to patients. Starting in March 2014 and ending in August 2015, Phase I of PCORnet was dedicated to establishing a governance model and building the distributed research network’s essential infrastructure that would enable the network to support large numbers of clinical trials and observational research studies during Phase II and beyond.

As part of this developmental phase, PCORI required an external evaluation to assess the implementation of the network during its first 18 months, including progress on network governance, data interoperability, streamlining of institutional review board (IRB) and research oversight, involvement of stakeholders, preparedness for observational studies and clinical trials, and other key markers of progress. Conducted by a team from RAND, the evaluation’s primary goals were to provide an objective assessment of PCORnet’s implementation, to gather and synthesize perspectives on key successes and challenges from PCORnet participants and stakeholders, and to characterize PCORnet’s overall “research readiness” by the end of Phase I.

This report presents the findings of this evaluation. The RAND team used data collected from ongoing observation of PCORnet meetings, stakeholder interviews, and reviews of progress reports and other administrative records from networks participating in PCORnet to conduct the evaluation. Quantitative data describing the development of PCORnet’s data infrastructure and other performance metrics were provided by the PCORnet Coordinating Center. We synthesized the information gathered from these sources to develop a cohesive picture of the status of the network as it neared the end of Phase I and to identify facilitators and barriers along the path to achieving a functioning research network.

The rest of this report is organized as follows: To provide necessary context, we begin by describing PCORnet’s foundations and the limitations of the existing research paradigm that PCORnet was designed to overcome. We then describe PCORnet’s participants, including the different types of research networks and the goals they sought to achieve in Phase I. Next, we

⁵ Patient Protection and Affordable Care Act, 2010.

describe our evaluation methodology and the framework we used to assess PCORnet's progress in three domains: implementation approach, process, and outcomes across 18 unique network activities. The rest of the report focuses on the progress of PCORnet's operational capacity, beginning with the development of a governance structure and the data and research infrastructures necessary to conduct multi-site observational research and clinical trials. We then discuss the extent and the nature of the collaborations between network partners over the course of Phase I, as well as PCORnet's efforts to engage patients and other stakeholders and lessons for ensuring ongoing engagement. In our concluding remarks, we highlight the key achievements during Phase I and opportunities for the continued evolution of the network in Phase II.

2. PCORnet's Foundations, Goals, and Participants

Major research innovations are often rooted in the need to address a gap or limitation in an existing research framework, a body of knowledge, or the technical capacity of a given field. In spite of the significant advances of medical science and health system research over the past several decades, the traditional research and dissemination paradigm has been (aptly) criticized as slow, expensive, and narrowly focused on “conditions” rather than patients.

PCORnet represents the culmination of federal, industry, and patient advocate efforts to change that paradigm and to usher in a new era of clinical research that upholds the tenets of “patient-centered” care. PCORnet seeks to address limitations in the current paradigm by expanding the role of patients and care providers in the research process and leveraging the expanded use of electronic health records and registries to conduct research that is cheaper, faster, and patient-centered in its focus and design. In the sections that follow, we describe the limitations of the existing research paradigm and the foundation of PCORnet in further detail, with an emphasis on the potential of the network to advance the science of clinical research.

Limitations of the Existing Research Paradigm

Since 1962, the U.S. Food and Drug Administration (FDA) has required drug and device manufacturers to provide specific clinical evidence in order to demonstrate the efficacy of their product prior to approval for market use. Marshaling this evidence requires clinical trials. Referred to as “efficacy research,” these trials are meant to ensure that the product is safe and provides “clinically significant results”⁶ for the target population. As the U.S. health system continues to grow, the demand for new products—and, therefore, clinical research—is rising. From 2010 to 2015, the number of U.S. clinical trials registered in the National Institutes of Health’s clinical studies database (clinicaltrials.gov) increased from over 51,000 to almost 88,000.⁷ Despite growth in the volume of clinical research, critics cite numerous flaws of the current research paradigm:

High cost and slow pace of clinical research. The slow pace of clinical research has been attributed to a host of factors, including lengthy IRB approval processes, extended periods of patient enrollment caused by low recruitment, and the multiple rounds of testing required to meet FDA efficacy standards. Because each of these steps must be completed sequentially, a delay at any point in the research process will ultimately delay the entirety of the clinical trial. Indeed, according to recent estimates from the Institute of Medicine (IOM), drug development can often

⁶ Basch, 2010.

⁷ ClinicalTrials.gov, undated.

take more than 10 to 15 years from research planning to market approval.⁸ As a result of the sluggish pace of clinical research, the cost of clinical trials has ballooned to over \$300 million⁹ per trial,¹⁰ with some estimates projecting that the total cost of bringing a new prescription drug to market averages \$2.6 billion.¹¹ The overall impact of the high cost and slow pace of research is ultimately borne by patients, who must not only wait years for new medical interventions to become available but must also pay high treatment costs once drugs come to market.

Lengthy patient recruitment. Further contributing to the length of clinical trials is the patient recruitment process.¹² To show subtle and meaningful treatment effects when identifying refinements in existing treatments, between 1,000 and 10,000 patients may be needed per trial,¹³ a sample size that requires significant time and effort to recruit. According to an estimate based on FDA data from 2006, meeting the demand for clinical trial participants would require one out of every 200 people in the United States to enroll in a trial,¹⁴ yet patient enrollment in clinical trials is low and continues to drop. As many as 27 percent of clinical trials fail to enroll any participants at all, upward of 75 percent of studies do not meet their enrollment targets, and 90 percent of studies need to extend the recruiting timeline, leading to delays.¹⁵

Limited information about subpopulations. Low patient enrollment exacerbates concerns related to the generalizability of study findings to patient subpopulations.¹⁶ This is often attributed to the nature of efficacy research itself: Clinical trials are performed in “ideal settings” that seek to reduce confounding variables (such as patient comorbidities) which could alter interpretations about a treatment’s efficacy. As a result, researchers apply strict inclusion and exclusion criteria¹⁷ that limit participation to a narrowly defined patient population that may not resemble the unselected patient populations typically seen in clinical settings. Although the clinical trial may describe the average effects of these interventions for study populations, the effects may not be representative for specific patient groups, such as racial and ethnic minorities, the elderly, women, and children.¹⁸ As a result, researchers are less able to identify or

⁸ English, Lebovitz, and Griffin, 2010.

⁹ English, Lebovitz, and Griffin, 2010.

¹⁰ English, Lebovitz, and Griffin, 2010.

¹¹ Tufts Center for the Study of Drug Development, 2014.

¹² V. Weisfeld, English, and Claiborne, 2012b.

¹³ English, Lebovitz, and Griffin, 2010.

¹⁴ English, Lebovitz, and Griffin, 2010.

¹⁵ English, Lebovitz, and Griffin, 2010.

¹⁶ Fleurence et al., 2013; Van Spall et al., 2007.

¹⁷ Van Spall et al., 2007.

¹⁸ Fleurence et al., 2013.

recommend optimal treatments for these subpopulations because of limited information on treatment effectiveness.

Minimal role of patients and other stakeholders in research. Patients play few roles other than study subjects in the traditional research paradigm.¹⁹ However, there is a growing consensus in the research field that stakeholder participation (including patients and patient advocates, clinicians, device manufacturers, and payers) is beneficial and that there is a need for patients and other stakeholder groups to participate in study design and research topic prioritization.²⁰ As the consumers or creators of medical services and products, patients and other stakeholders are uniquely positioned to identify new opportunities or growth areas in health research and to help guide the development of a health system that meets the full spectrum of patient needs.

Low participation of community physicians in clinical research. The limited role that community physicians currently play in clinical research has been identified as a key factor that contributes to the divide between research and clinical care.²¹ Barriers limiting the participation of community physicians in research include the need to balance the additional administrative burden of research with the ongoing demands of running a practice, as well as a lack of financial and administrative support from within the research infrastructure.²² In addition, community physicians may lose revenue by referring their patients elsewhere for trials.²³

The result of the overall lack of community physician involvement in research is threefold: (1) Fewer physicians refer patients to clinical studies, (2) the needs and priorities of community physicians are less likely to be represented in new research studies, and (3) community physicians may be less likely to adopt research findings generated from academic medical centers.²⁴

Unrealized opportunities to leverage clinical data captured in electronic health records, registries, and other sources. Multiple opportunities exist to leverage the collective power of data captured in electronic health records (EHRs), registries, and other patient-oriented tools for clinical research. To date, however, these opportunities are largely unrealized because of data systems that lack interoperability, local rather than universal data definitions or structures, and limited infrastructure for sharing health data in ways that provide strong patient privacy safeguards. As health information increasingly shifts to electronic rather than paper-based formats, proponents argue that solutions are at hand: Existing data from payers, EHRs, health

¹⁹ English, Lebovitz, and Griffin, 2010; Engleberg Center for Health Care Reform at Brookings, 2014.

²⁰ English, Lebovitz, and Griffin, 2010; Fleurence et al., 2013; Engleberg Center for Health Care Reform at Brookings, 2014; PROTaskForce, 2014.

²¹ V. Weisfeld, English, and Claiborne, 2012b.

²² English, Lebovitz, and Griffin, 2010.

²³ English, Lebovitz, and Griffin, 2010.

²⁴ English, Lebovitz, and Griffin, 2010.

care delivery systems, and condition-tracking tools present an opportunity to facilitate low-cost, large-scale definitive trials, reduce expenses related to clinical data collection, and shorten the amount of time it takes to conduct research.²⁵

Emerging Opportunities to Create a New Research Paradigm

Overcoming these limitations on the quality and use of clinical research will become increasingly important in the United States as the demographic makeup of the country shifts and the health information technology sector continues to innovate. Indeed, as the United States grows older and more diverse²⁶ and produces an increasing amount of administrative and patient-reported health data, identifying timely and targeted treatments that account for emerging care needs, comorbidities, and patient preferences will be critical for optimizing patient and population health.

PCORnet's Vision and Goals

Envisioning PCORnet

The idea of PCORnet emerged as a response to these challenges and as a way forward in transitioning toward a new patient-centered research paradigm. PCORnet is meant to combine resources and strategies so as to create a multiplier effect in health and health systems research. By leveraging relationships and partnerships between organizations, developing a data infrastructure to support data-sharing, cultivating a patient-centered research focus, and enhancing the visibility and utility of comparative effectiveness research (CER), PCORnet aims “to respond to [patient] priorities and speed the creation of new knowledge to guide treatment on a national scale.”²⁷

The centerpiece of the PCORnet initiative is the design and implementation of a distributed research network (DRN) that combines patient-generated and clinical data.²⁸ A DRN and “distributed querying” allow a spectrum of analyses to be conducted while maintaining data safety and security under the protection of institutional firewalls, subsequently minimizing or eliminating the risk of releasing protected health information.²⁹ PCORnet’s DRN framework is intended to facilitate multi-site interventional and observational research among its participants

²⁵ Collins, 2012.

²⁶ According to recent Census estimates, the number of individuals aged 65 and over will increase from 43.1 million in 2012 to over 83 million in 2050, including nearly 9 million aged 85 and over. During this same period, the Hispanic population is projected to double, and the number of individuals identifying as more than one race is expected to increase from 333,000 to 1.5 million (Ortman, Velkoff, and Hogan, 2014).

²⁷ PCORnet, undated(i).

²⁸ Engleberg Center for Health Care Reform at Brookings, 2014.

²⁹ Fleurence, Curtis, et al., 2014b.

and will help facilitate rapid-learning research, which is central to CER.³⁰ Unlike a centralized database that requires data sharing, a DRN allows individual networks to design data files in a standard format called a common data model, and networks maintain possession of their data files.³¹ The DRN and common data model allow participants to query data across PCORnet while aligning with individual network data privacy, patient security, institutional confidentiality, and governance needs.³²

The initiative envisions that both PCORnet researchers and researchers outside of the network will use PCORnet's DRN³³ to bolster research on a variety of health problems for which there are otherwise few opportunities or about which there is limited research. With its patient-centered focus, PCORnet would provide a unique resource in which researchers can address questions that are relevant to patients seeking information about their health care options and receive answers more quickly than under the current research paradigm. Moreover, the distributed networking platform is shared with the NIH Health Care Systems Research Collaboratory,³⁴ the FDA's Mini-Sentinel program,³⁵ and the HMO Research Network,³⁶ raising the potential for future linkages between PCORnet and these other networks in the future.

PCORnet Participants and Stakeholders

In December 2013, PCORI's Board of Governors authorized funding for 11 Clinical Data Research Networks (CDRNs) and 18 Patient-Powered Research Networks (PPRNs) to become the initial networks comprising PCORnet and to develop the infrastructure for the distributed research network. In addition, PCORI funded a Coordinating Center whose role was to oversee critical elements of PCORnet's development, provide technical assistance, and address important

³⁰ Engleberg Center for Health Care Reform at Brookings, 2014; N. Weisfeld, English, and Claiborne, 2012a.

³¹ Fleurence, Curtis, et al., 2014b; Behrman et al., 2011.

³² Brown et al., 2010.

³³ Chen et al., 2014.

³⁴ The National Institutes of Health (NIH) funded the Health Care Systems Research Collaboratory in 2012 to improve the clinical trials system in the United States by addressing barriers in biomedical research that impede basic scientific discovery and its translation into improved human health. The Collaboratory's distributed research network is designed to facilitate multi-site pragmatic clinical trials (NIH Collaboratory, undated).

³⁵ Harvard Pilgrim Health Care Institute received funding from the FDA in 2009 to create a national electronic system for monitoring the safety of FDA-regulated medical products using medical claims data, patient registries, and inpatient and outpatient medical records. The Mini-Sentinel program was the pilot program and consisted of a distributed data network comprised of electronic health information for more than 60 million people (Mini-Sentinel, 2014).

³⁶ The National Cancer Institute's Cancer Research Network established the HMO Research Network (HMORN) Virtual Data Warehouse in 2003. The HMORN (now known as the Health Care Systems Research Network) combines EHR data for about 16.5 million people across 19 health systems to enable population-based research in real-world settings.

aspects of the research process to facilitate the establishment of the network during its first 18 months.³⁷ The leadership of PCORnet consisted of three unique entities: the Steering Committee, its Executive Committee, and the Patient Council.³⁸ Descriptions of each of these participant groups and their primary activities during Phase I follow. Chapter 4, “Developing and Implementing PCORnet’s Governance Infrastructure,” includes a discussion of the evolution of these entities.

Clinical Data Research Networks

A CDRN is a collaboration of health systems tasked with identifying patient cohorts and creating, maintaining, and standardizing electronic resources to support PCORnet research activities. Consisting of health systems that include academic health centers, acute care providers, safety net clinics, integrated delivery systems, and a regional health information exchange,³⁹ the 11 CDRNs participating in Phase I are data-focused entities whose partnerships are designed to facilitate large, comprehensive, and longitudinal electronic clinical and health data systems (Table 2.1).⁴⁰ CDRNs are located in diverse regions of the United States, and the number of patients seeking care within the delivery systems comprising each CDRN ranged from 1 million to 28 million patients.

Most CDRNs had substantial experience with data warehouses and performing extract, transform, and load⁴¹ (ETL) processes as part of their previous work with collaborative data-sharing initiatives that preceded PCORnet. Specifically, several respondents reported playing significant development or support roles in the creation of the Informatics for Integrating Biology and the Bedside⁴² (i2b2) initiative, the Observational Medical Outcomes Partnership,⁴³ the HMO Research Network Virtual Data Warehouse, and the Mini-Sentinel program. As a result of this experience, CDRN participants had sophisticated data models and processes in place that could be adapted for use in PCORnet. In fact, some CDRN partners were primarily

³⁷ Fleurence, Curtis, et al., 2014b; Fleurence, Beal, et al., 2014a.

³⁸ Fleurence, Curtis, et al., 2014b; Engleberg Center for Health Care Reform at Brookings, 2014.

³⁹ Fleurence, Curtis, et al., 2014b.

⁴⁰ Fleurence, Curtis, et al., 2014b.

⁴¹ ETL “is a process in which programmers extract data from one or more data sources, transform the data to fit certain requirements or specifications, and then load the data into a desired location. In the context of PCORnet, CDRN and PPRN database programmers extract the data needed to populate the PCORnet common data model from the data sources which house the necessary information, transform their data to fit into the common data model, and then load that transformed data into a defined location” (PCORnet Central Desktop, undated).

⁴² i2b2 is an NIH-funded National Center for Biomedical Computing based at Partners HealthCare System. The i2b2 Center developed a common data model to facilitate use of EHR data to inform patient care.

⁴³ The Observational Medical Outcomes Partnership (OMOP) is a public-private health informatics partnership that maintains a common data model that is used by its member community to conduct observational research on electronic health care databases (OMOP, undated).

responsible for creating and beta testing the common data model that ultimately served as the foundation of the PCORnet data model (for more information about the common data model, see Chapter 5, “Developing PCORnet’s Data Infrastructure.”

Along with the varying levels of experience in developing and using patient data models, CDRNs also reported a wide range of experience in collecting patient-generated data prior to Phase I. Roughly half of the CDRN respondents noted that they were collecting such data prior to Phase I; however, most variables were collected for the purpose of monitoring routine care or for specific clinical studies and were not systematically collected for broader research purposes. Additionally, these data typically did not include patient-reported outcomes (PROs).

Table 2.1. Clinical Data Research Networks Participating in Phase I

Network Name	Organization Type(s)	Geography	Population covered
Accelerating Data Value Across a National Community Health Center Network (ADVANCE)	Network of low-income clinics	National	2 million
Chicago Area Patient Centered Outcomes Research Network (CAPriCORN)	Community trust, health systems in large urban area	Midwest	5.5 million
Great Plains Collaborative (GPC)	Academic medical centers	Midwest	12 million
Kaiser Permanente & Strategic Partners Patient Outcomes Research To Advance Learning (PORTAL) Network	Integrated health systems	National	10.5 million
Louisiana CDRN (LACDRN)	Health information exchange (HIE)-based	South	1 million
Mid-South CDRN	Academic medical centers	South	28 million
National Pediatric Learning Health System (PEDSNet)	Children's hospital consortium	National	2 million
New York City Clinical Data Research Network (NYC-CDRN)	Community trust, health systems in large urban area	Northeast	10.5 million
PaTH: Towards a Learning Health System in the Mid-Atlantic Region	Academic medical centers	Mid-Atlantic	2.5 million
Patient-oriented SCALable National Network for Effectiveness Research (pSCANNER)	Academic medical center and Veterans Affairs health system	West Coast	22 million
Scalable Collaborative Infrastructure for a Learning Healthcare System (SCILHS)	Academic medical center	Northeast	8 million

Patient-Powered Research Networks

One of PCORnet’s main goals is to establish communities in which patients form partnerships with researchers.⁴⁴ To this end, PCORnet invited Patient-Powered Research Networks (PPRNs) to participate in PCORnet’s development. PPRNs comprise patients, caregivers, and families who are “motivated to build an ideal network [of stakeholders] and play an active role in patient-centered comparative effectiveness research.”⁴⁵ Most of the 18 PPRNs

⁴⁴ Fleurence, Curtis, et al., 2014b.

⁴⁵ PCORI, 2013a.

participating in Phase I consisted of individuals who were motivated by a shared medical condition and highly engaged patient communities to form a network designed to advance the state of science by incorporating stakeholder voices into the research process. In collaboration with CDRNs and other PCORnet stakeholders, patients in PPRNs are helping to generate research questions, contributing to the data-sharing process by collecting and disseminating PROs and other patient-generated data. They are also encouraged to get involved in interventional trials and are invited to help interpret and share study results.⁴⁶ PPRNs are also focused on expanding the size of their networks and are committed to enhancing standardized data collection.⁴⁷

The PPRNs funded in Phase I represent a broad diversity of health conditions and range from established patient advocacy organizations to newly formed networks of research groups and patient communities (Table 2.2). PCORI selected nine common-condition PPRNs, eight rare-condition PPRNs, and one network focusing on both common and rare conditions to develop PCORnet—recognizing the need to advance research in both areas. As a result of this diversity, PPRNs also reported a wide range of experience in collecting patient-generated data prior to Phase I. Whereas some PPRNs have advanced data capabilities, such as longitudinal disease registries and online PRO tracking applications (see Chapter 5, “Developing PCORnet’s Data Infrastructure,” for more information about PRO data collected by CDRNs and PPRNs), other organizations are less advanced. Similarly, some PPRNs have active research programs, while others are relatively new to research.

⁴⁶ Fleurence, Curtis, et al., 2014b.

⁴⁷ Fleurence, Curtis, et al., 2014b.

Table 2.2. Patient-Powered Research Networks Participating in Phase I

PPRN	Condition
Common-condition PPRNs	
American BRCA Outcomes and Utilization of Testing Patient-Powered Research Network (ABOUT Network)	Hereditary breast and ovarian cancer
ARthritis patient Partnership with comparative Effectiveness Researchers (AR-PoWER PPRN)	Arthritis (rheumatoid arthritis, spondyloarthritis), musculoskeletal disorders (osteoporosis), and inflammatory conditions (psoriasis)
CCFA Partners Patient-Powered Research Network	Inflammatory bowel disease (Crohn's disease and ulcerative colitis)
The COPD Patient-Powered Research Network	Chronic obstructive pulmonary disease
The Health eHeart Alliance	Cardiovascular health
ImproveCareNow: A Learning Health System for Children with Crohn's Disease and Ulcerative Colitis	Pediatric Crohn's disease and ulcerative colitis
Mood Patient-Powered Research Network	Major depressive disorder and bipolar disorder
Multiple Sclerosis Patient-Powered Research Network	Multiple sclerosis
Sleep Apnea Patient-Centered Outcomes Network (SAPCON)	Sleep apnea
Rare-condition PPRNs	
ALD Connect	Adrenoleukodystrophy
The DuchenneConnect Patient-Report Registry Infrastructure Project	Duchenne and Becker muscular dystrophy
NephCure Kidney Network for Patients with Nephrotic Syndrome	Primary nephrotic syndrome (focal segmental glomerulosclerosis, minimal change disease, and membranous nephropathy)
Patients, Advocates and Rheumatology Teams Network for Research and Service (PARTNERS) Consortium	Juvenile rheumatic disease
Phelan-McDermid Syndrome Data Network	Phelan-McDermid syndrome
PI Patient Research Connection (PI-CONNECT)	Primary immunodeficiency diseases
Rare Epilepsy Network (REN)	Aicardi syndrome, Lennox-Gastaut syndrome, Phelan-McDermid syndrome, hypothalamic hamartoma, Dravet syndrome, and tuberous sclerosis
The Vasculitis Patient-Powered Research Network	Vasculitis
Rare and Common Condition PPRNs	
Community-Engaged Network for All (CENA)	Alström syndrome, Dyskeratosis congenital, Gaucher disease, hepatitis, inflammatory breast cancer, Joubert syndrome, Klinefelter syndrome and associated conditions, metachromatic leukodystrophy, pseudoxanthoma elasticum (PXE), psoriasis

Coordinating Center

The Coordinating Center is the PCORnet entity primarily responsible for ensuring that PCORnet achieves its organizational and strategic goals.⁴⁸ Specifically, the Coordinating Center is charged with providing technical and logistical support to CDRNs and PPRNs and “plays a critical role in fostering communication and coordination . . . as well as disseminating best

⁴⁸ Engleberg Center for Health Care Reform at Brookings, 2014.

practices.”⁴⁹ The Coordinating Center is made up of two entities: the task forces and the Project Management Office (PMO),⁵⁰ and it works with PCORnet’s Steering Committee (one of PCORnet’s governance bodies, discussed below) to put Steering Committee recommendations into practice.

The Coordinating Center itself is led by the Harvard Pilgrim Health Care Institute, along with Duke University and the Genetic Alliance.⁵¹ The following groups also partnered with the Coordinating Center for all or Part of Phase I: AcademyHealth, the Brookings Institution, the Center for Medical Technology Policy, the Center for Democracy & Technology, the Group Health Research Institute, the Johns Hopkins Berman Institute of Bioethics, and America’s Health Insurance Plans.⁵²

Task Forces

In order to “address important aspects of the research process” during Phase I, the Coordinating Center developed 11 task forces designed to help “identify effective practices and build common solutions for more efficient multi-site distributed research.”⁵³ Composed of PPRN and CDRN members who were nominated to represent their respective networks, the task forces identified priority topics, tasks, or activities that guided the task force’s work during Phase I. In general, priorities were divided into three basic domains: process and management guidance related to the development of infrastructure within PCORnet, the creation of specific deliverables that facilitated networks’ infrastructure-building activities, and collaborations or partnerships with groups external to PCORnet. Most task forces ended their activities after the first year of Phase I, as described in Chapter 4, “Developing and Implementing PCORnet’s Governance Infrastructure.”

For a description of each task force, see the appendix.

Project Management Office

The purpose of the PMO is to “oversee the core functions of Program Management, Technical Assistance, Cross Awardee Activities, Evaluation and Logistical Support.”⁵⁴ Specifically, the PMO supervises, coordinates, and supports efforts surrounding best practices,

⁴⁹ Fleurence, Curtis, et al., 2014b; Engleberg Center for Health Care Reform at Brookings, 2014; PCORI, 2014.

⁵⁰ Engleberg Center for Health Care Reform at Brookings, 2014.

⁵¹ The Genetic Alliance became part of the Coordinating Center leadership after the first year of Phase I. Fleurence, Curtis, et al., 2014b.

⁵² PCORnet, undated(i).

⁵³ PCORnet, undated(n).

⁵⁴ Engleberg Center for Health Care Reform at Brookings, 2014.

policy-sharing, infrastructure related to information-sharing, and technical assistance to the task forces.⁵⁵

Steering Committee

The Steering Committee includes clinician and patient representatives from each CDRN and PPRN, as well as representatives from stakeholder groups, including NIH, FDA, the Agency for Healthcare Research and Quality (AHRQ), Centers for Disease Control and Prevention (CDC), the Centers for Medicare & Medicaid Services (CMS), the Office of the National Coordinator for Health Information Technology (ONC), and the Office of the Assistant Secretary for Planning and Evaluation (ASPE) of the U.S. Department of Health and Human Services. The Steering Committee also receives input from industry representatives, including medical product and device manufacturers and the health insurance industry.⁵⁶

Executive Committee

The Executive Committee is designated by the Steering Committee to develop strategies and processes for PCORnet implementation around specific topics to support decisionmaking by the larger Steering Committee. The Executive Committee is composed of the Steering Committee chair, representatives from two CDRNs and two PPRNs, the PCORI executive director (or designee), and the Coordinating Center director and co-director.

Patient Council

The PCORnet Patient Council is a deliberative body comprising seven individuals who “provides feedback and recommendations on key PCORnet policies to ensure full consideration of both the highest patient engagement standards and issues related to protection of patient privacy, consent, and autonomy.”⁵⁷ Members include patients and caregivers and PCORI’s director of patient engagement. The Patient Council conducted its activities through the end of Phase I, at which point responsibility for overseeing patient engagement within PCORnet was transferred to a work group under the direction of the Executive Committee.

How PCORnet Addresses Limitations of the Existing Research Paradigm

In order to address the problems with the existing research paradigm, the networks that make up PCORnet are intended to engage patients, family members, caregivers, and other health care

⁵⁵ Engleberg Center for Health Care Reform at Brookings, 2014.

⁵⁶ Fleurence, Curtis, et al., 2014b.

⁵⁷ PCORI, 2013b.

stakeholders in every aspect of the research and dissemination process.⁵⁸ Because these stakeholders are often the ones who bear the brunt of the impacts of slow, costly, and narrowly focused clinical research, incorporating a greater diversity of perspectives and voices should add both richness and urgency to the PCORnet research process. Specifically, stakeholder engagement is expected to contribute to improved data privacy and security standards, the prioritization of research agendas that matter to patients, an increase in the broad and diverse patient populations participating in research studies, dissemination of research results, and the management and leadership of the networks.⁵⁹

In particular, PPRNs are especially focused on patient engagement, and many have already been working with health advocacy organizations to enhance the diversity within their patient communities.⁶⁰ Additionally, the PPRNs are designed to be “learning networks” in which the type of research and data collected will be purposeful and is collected with the intent of improving patient outcomes.⁶¹ PPRN efforts geared toward improving patient participation in research will be founded on transparency surrounding risks as well as potential benefits.⁶² Lastly, the vision for PPRNs is to develop their governance structures with the aims of including patients in leadership positions and designing operational policies to ensure patient inclusion in developing research agendas.

The combination of a robust data network/sharing structure represented by CDRNs and enhanced stakeholder engagement has the potential to lead to observational trials and randomized studies that could be designed and implemented more quickly and at a much lower expense than for current trials.⁶³ In addition to the lower cost and faster pace of research, the wide scope of EHR data has the potential to capture data from a much larger number of individuals in subpopulations, therefore increasing their visibility in health outcomes studies. This visibility could be further enhanced by direct participation in clinical trials because of the cultivation of strong relationships among patient groups of interest. Finally, if the barriers to community physician participation (cost, administrative burden) are reduced, their increased participation would not only enrich the overall perspective of practitioners in the clinical research field but would also increase the likelihood of the implementation of evidence-based practices based on PCORnet research.

⁵⁸ Fleurence, Beal, et al., 2014a.

⁵⁹ Fleurence, Beal, et al., 2014a.

⁶⁰ Fleurence, Beal, et al., 2014a.

⁶¹ Fleurence, Beal, et al., 2014a.

⁶² Fleurence, Beal, et al., 2014a.

⁶³ Collins, 2012.

PCORnet’s Phase I Goals and Participant Requirements

PCORnet’s Phase I activities were guided by a set of 18-month aims developed in the early stages of Phase I and a set of requirements for CDRNs and PPRNs that were specified in funding announcements specific to each network that were released in fall 2013. The 18-month aims (see Table 2.3) focused on discrete targets that aligned stakeholder groups in order to bring together “expertise, populations, resources, and data” and build the foundation for a national patient-centered research infrastructure.⁶⁴

Table 2.3. PCORnet’s 18-Month Aims

Highly engaged patients, clinicians, health systems, researchers, and other partners
A collaborative community
Analysis-ready standardized data with strong privacy and data security protections
Oversight that protects patients, supports timely conduct of research, and builds trust in the research enterprise
Research integrated into care settings and with patient communities

In order to achieve the 18-month aims, PCORnet would leverage knowledge from existing distributed research networks (such as the FDA’s Mini-Sentinel program and NIH’s Health Care Systems Research Collaboratory), as well as other efforts, such as AcademyHealth’s Electronic Data Methods⁶⁵ (EDM) Forum for Comparative Effectiveness Research.⁶⁶ PCORnet would also facilitate stakeholder interactions that promote a “culture of trust” and would develop a uniform governance infrastructure that promotes consistency across all stakeholder groups.⁶⁷ PCORI was acutely aware of the challenges in developing trust between entities around issues such as sensitive patient data and information on the performance of individual health systems.⁶⁸ The 18-month aims were designed to build a strong foundation for PCORnet’s long-term success.

An additional goal that emerged early in the course of Phase I was for the network to design and launch both a large, multi-site clinical trial and a large observational study that leveraged PCORnet’s emerging infrastructure. These demonstration projects would give participants a way to engage with one another in diverse activities with common short-term goals and to strengthen

⁶⁴ Fleurence and Selby, 2014.

⁶⁵ AcademyHealth’s EDM Forum was established in 2010 with support from AHRQ to facilitate collaboration between researchers and stakeholder groups involved with generating data, methods, and evidence and seeks to accelerate the translation and dissemination of health care innovations (AcademyHealth, undated).

⁶⁶ PCORnet, undated(i).

⁶⁷ Engleberg Center for Health Care Reform at Brookings, 2014.

⁶⁸ Vest and Gamm, 2010.

learning opportunities. Moreover, the successful launch and, ultimately, the completion of these studies would provide compelling evidence to potential funders or users of PCORnet. We describe the three key demonstration projects launched during Phase I briefly below.

- **Aspirin Dosing: A Patient-Centric Trial Assessing Benefits and Long-term Effectiveness (ADAPTABLE).** Aspirin Dosing: A Patient-Centric Trial Assessing Benefits and Long-term Effectiveness (ADAPTABLE) is a pragmatic, patient-level randomized clinical trial that seeks to enroll 20,000 patients and to “identify the optimal dose of aspirin for secondary prevention in atherosclerotic cardiovascular disease.” The trial will use streamlined identification and recruitment approaches, including electronic consent and data collection. During Phase I, the topic was selected by consensus by the PCORnet Steering Committee and approved by the PCORI Board of Governors, the research protocol was drafted, \$14 million in funding was allocated, and planning for the trial commenced.⁶⁹ Current activities are focusing on patient enrollment with hopes of achieving 50-percent enrollment by November 2016.
- **Short- and Long-Term Effects of Antibiotics on Childhood Growth,** the first of two large-scale observational studies being undertaken during Phase I, will utilize EHR data generated from nine CDRNs to identify relationships between antibiotic use in early childhood and body mass indexes (BMI) and/or growth trajectories at ages 5 and 10. The primary objective of the study is to understand how antibiotic use could affect BMI and growth, to understand the significance of any differences between specific subgroups of the population, and to explore ways to integrate these findings into everyday clinical practice.
- **The PCORnet Bariatric Study** is designed to fill existing gaps in clinical knowledge about the “optimal choice of bariatric surgical procedure in various populations” and to identify patient preferences and motivations for bariatric surgery. The risks and outcomes of the three most common bariatric procedures will be evaluated using EHR data from ten CDRNs (which include over 60,000 patients and 900 adolescent bariatric patients), as well as focus groups with patients and other stakeholders. The primary objective of the study is to understand subtle differences in patient preferences and health outcomes across specific patient subgroups, including older adults, racial/ethnic minorities, and adolescents, to better inform patient and provider decisions about the choice of surgical procedure best suited to meet patient needs.

PCORnet participants who are engaged in one or both observational studies have begun to identify research questions through a process that has engaged diverse stakeholders (including patients) during the spring and summer of 2015. As of September 2015, the research teams are

⁶⁹ PCORI, 2015a.

finalizing their research plans. After executing all subcontracts and obtaining IRB approval, the teams will identify the final study cohorts.⁷⁰

While participating in these demonstration projects was optional, CDRNs and PPRNs faced a core set of Phase I requirements that were listed in their respective funding announcements (Table 2.4). Notably, CDRNs were required to standardize data on a population of at least 1 million patients, enroll patients into three unique condition cohorts and survey these patients, and engage a diverse set of stakeholders. PPRNs were expected to reach specific enrollment targets, collect data from patients, standardize data, and engage patients in the governance of their local network.

Table 2.4. CDRN and PPRN Phase I Requirements

CDRN	PPRN
<ul style="list-style-type: none"> • 1 million patients enrolled • At least three patient cohorts identified, characterized, and surveyed • EHR standardized within collaboration network • Network’s data in standardized, interoperable format with other awardee networks • Capable of implementing clinical trials • Patients, systems, and clinicians engaged in governance and use of network 	<ul style="list-style-type: none"> • Recruit 0.5 percent of U.S. population with condition (minimum of 50 patients for rarest of diseases and minimum of 50,000 for most common conditions) • Patient-reported data collected for at least 80 percent of cohort • Standardized data suitable for PCORnet research • Patients fully involved in network governance

The PCORI Board of Governors’ Phase I startup investment for infrastructure development and operations was \$102 million.⁷¹ One of the primary challenges from the beginning was to plan for long-term sustainability.⁷² At the start of Phase II (beginning in September 2015), PCORI began tapering its infrastructure funding,⁷³ and PCORnet’s sustainability from Phase II onward is expected to be supported by multiple funding sources—in particular, competitive research funding (e.g., specific research projects funded by PCORI and federal funders, such as NIH, FDA, and pharmaceutical and medical device manufacturers⁷⁴).

It is also anticipated that as PCORnet matures and its funding opportunities widen, the network will open more broadly to external researchers and external data partners⁷⁵ interested in contributing to CER and patient-centered research.

⁷⁰ PCORI, 2015b.

⁷¹ Fleurence, Beal, et al., 2014a.

⁷² Fleurence, Beal, et al., 2014a.

⁷³ Fleurence and Selby, 2014.

⁷⁴ Fleurence and Selby, 2014.

⁷⁵ Fleurence, Curtis, et al., 2014b.

The next chapter provides details of RAND's evaluation methodology that was used to assess PCORnet's implementation progress in Phase I relative to the goals and requirements that PCORI established for the network.

3. Evaluation Methodology

Goals of the External Evaluation

PCORI contracted with RAND to conduct an external evaluation to provide an objective and comprehensive assessment of PCORnet's implementation progress during Phase I. The type of evaluation selected for Phase I was a formative evaluation, which is designed to identify early successes, challenges, and areas of concern that can help improve a policy or program's implementation and increase the likelihood that it achieves its desired outcomes.

Accordingly, the three key goals of the external evaluation were as follows:

1. Identify the key accomplishments and challenges implementing PCORnet within key activity areas.
2. Gather information on the experience of PCORnet participants and stakeholders and their perceptions of implementation progress during Phase I.
3. Synthesize available information to assess the extent to which PCORnet was on the path to research readiness by the end of Phase I.

The external evaluation was designed to complement three other evaluation activities led by PCORI or other PCORnet entities.

- First, PCORI program officers monitored CDRN and PPRNs' compliance with all contractual milestones for which CDRNs and PPRNs were required to submit supportive documentation.
- Second, PCORI conducted a patient engagement evaluation that drew primarily on the results of the PCORnet Engagement Activity Inventory (netENACT) survey, which was administered every six months to CDRNs and PPRNs. The goal of the netENACT survey was to provide a standardized method of assessing the level and nature of stakeholder engagement during Phase I with a focus on patient involvement in PCORnet activities.
- Third, the PCORnet Executive Committee led the ongoing monitoring of performance of individual networks using data collected by the Coordinating Center or by the Executive Committee through periodic data requests made to CDRNs and PPRNs.

The external evaluation was designed to avoid redundancy with these other evaluation efforts. In particular, the focus of the external evaluation was to assess the progress of PCORnet as a whole rather than to evaluate individual CDRNs or PPRNs. The external evaluators did not contribute to PCORI's decisions to continue or discontinue funding to individual networks, either as part of PCORI's own six-month evaluation or PCORI's Phase II funding decisions. Furthermore, given the high data collection burden placed on CDRNs and PPRNs to support

other evaluations and other Phase I activities (including data requests that supported the work of the task forces), RAND and PCORI determined early in Phase I that the external evaluation would not undertake new data collection efforts, with the exception of a comprehensive set of stakeholder interviews (described below). Rather, the external evaluation would leverage the data collected to support these other evaluations to the extent possible. Reliance on existing data sources was considered a practical solution to the need for performance data that minimize the burden on participants.

Evaluation Framework

We developed an evaluation framework to assess PCORnet’s implementation progress by drawing on components of implementation evaluation frameworks available in the literature. Particularly useful were frameworks published by the U.S. Government Accountability Office (GAO)⁷⁶ and the Substance Abuse and Mental Health Services Administration (SAMHSA)⁷⁷ that suggested three key dimensions for evaluating the implementation of a program: (1) whether the program is implemented as intended, (2) the extent and nature of any challenges that emerge during the program’s implementation and the extent to which refinements to the program are made to address these challenges, and (3) the degree to which the program achieves its expected outcomes—including participants’ perceptions of the program’s implementation. In the remainder of this section, we describe the three evaluation domains comprising our evaluation framework that combine the essential components of these other frameworks.

Implementation Approach. This domain examines whether structures were in place to guide implementation activities and helps to assess whether implementation of specific components of PCORnet occurred as intended. We examined the extent to which one or more groups were assigned to lead or coordinate specific network activities, the specification of goals linked to each network activity, and the development of concrete plans to achieve each goal. Where relevant, we examined the roles played by different stakeholder groups in the implementation process.

Implementation Process. The second evaluation domain focuses on the implementation challenges that may have arisen in the course of executing PCORnet’s implementation plans. We examined the extent to which participants overcame early implementation challenges, whether through assistance provided by other participants, the Coordinating Center, or other means, and

⁷⁶ The GAO publication *Designing Evaluations* describes an audit and evaluation methodology framework derived from GAO studies and policy documents, as well as GAO program evaluation literature (GAO, 2012).

⁷⁷ The SAMHSA resource “Using Process Evaluation to Monitor Program Implementation” provides a process evaluation framework for analyzing program activity delivery in order to assess program implementation quality (SAMHSA, 2015).

both the barriers and facilitators of success in overcoming these challenges, from the perspective of diverse stakeholder groups.

Implementation Outcomes. This domain includes an assessment of the extent to which the implementation plan was successfully executed and the Phase I goals were achieved. We also include participants' overall experience during the implementation process because this information may help to evaluate the level of network cohesion, the willingness of funders to invest in PCORnet, or other factors that may impact PCORnet's longer-term success.

Five Domains of Network Activities

The evaluation framework was used to examine PCORnet's implementation progress within 18 specific "network activities." These activities were developed by the evaluation team through a review of PCORnet funding announcements and observation of Steering Committee and task force meetings. These activities represent the areas in which PCORnet participants were expected to devote most of their efforts during Phase I. The evaluation team then grouped the network activities into five higher-level implementation domains: PCORnet's governance infrastructure, PCORnet's data infrastructure, PCORnet's research infrastructure, PCORnet-level collaboration, and stakeholder engagement. These domains were designed to provide greater structure to the evaluation and to facilitate the synthesis of findings across related implementation activities.

Table 3.1 displays the five implementation domains, the network activities that map to each implementation domain, and the topics developed by the evaluation team that addressed each of three domains of the evaluation framework. Evaluation topics for the implementation approach and implementation process domains were derived inductively using the framework; implementation outcomes topics were taken from funding announcements and other documents or were derived over time as implementation priorities emerged.

Table 3.1. External Evaluation Framework

Network Activity	Implementation Approach	Implementation Process	Implementation Outcomes
PCORnet's Governance Infrastructure			
Decisionmaking	<ul style="list-style-type: none"> Stakeholder roles in decisionmaking (PCORI, Coordinating Center, CDRNs/PPRNs) 	<ul style="list-style-type: none"> CDRN/PPRN participation Timeliness of decisionmaking 	<ul style="list-style-type: none"> Stakeholder perceptions of decisionmaking Responsiveness to issues
Developing PCORnet policies	<ul style="list-style-type: none"> Roles of governance entities (PCORnet Council, Executive Committee, Coordinating Center, Patient Council) 	<ul style="list-style-type: none"> Participation in policy development Timeliness of policy development 	<ul style="list-style-type: none"> Stakeholder experience participating in policy development
Communication and coordination	<ul style="list-style-type: none"> Coordinating Center communication infrastructure 	<ul style="list-style-type: none"> Coordination between task forces and between task forces and CDRNs/PPRNs 	<ul style="list-style-type: none"> Stakeholder perception of timeliness and clarity of communication
PCORnet's Data Infrastructure			
Standardizing data	<ul style="list-style-type: none"> Coordinating Center approach to common data model development/updating 	<ul style="list-style-type: none"> Key implementation challenges Strategies used to address challenges 	<ul style="list-style-type: none"> Extent of standardization within CDRN/PPRN DataMarts
Developing querying capability	<ul style="list-style-type: none"> Coordinating Center approach to implementing query tool 	<ul style="list-style-type: none"> Key implementation challenges Strategies used to address challenges 	<ul style="list-style-type: none"> CDRNs and PPRNs successfully respond to test query
Enhancing data quality/ completeness	<ul style="list-style-type: none"> Coordinating Center/CDRN/PPRN plan to assess data quality Coordinating Center/CDRN/PPRN plan to achieve complete data 	<ul style="list-style-type: none"> Key implementation challenges Strategies used to address challenges 	<ul style="list-style-type: none"> Level of data quality as assessed through queries Completeness of longitudinal data
Protecting patients' privacy	<ul style="list-style-type: none"> Development of guidance on data privacy standards 	<ul style="list-style-type: none"> Key implementation challenges Strategies used to address challenges 	<ul style="list-style-type: none"> Data privacy protections in place
Collecting patient-generated data	<ul style="list-style-type: none"> PCORnet process for selecting PROs to include in common data model 	<ul style="list-style-type: none"> Key implementation challenges Strategies used to address challenges 	<ul style="list-style-type: none"> Extent of data collection for PROs in common data model
Developing biobanking capabilities	<ul style="list-style-type: none"> Plan to expand biospecimen collection Plan to facilitate use of biospecimens in PCORnet research 	<ul style="list-style-type: none"> Key implementation challenges Strategies used to address challenges 	[Not considered during Phase I because it was a lower priority]

Network Activity	Implementation Approach	Implementation Process	Implementation Outcomes
PCORnet's Research Infrastructure			
Streamlining IRB review	<ul style="list-style-type: none"> CDRN selection of streamlined IRB model 	<ul style="list-style-type: none"> Key implementation challenges Strategies used to address challenges 	<ul style="list-style-type: none"> CDRN IRBs able to review protocols for CDRN research CDRN early experience with streamlined IRB models PPRN IRB approval
Enhancing informed consent processes	<ul style="list-style-type: none"> CDRN/PPRN plans for enhancing informed consent process 	<ul style="list-style-type: none"> Key implementation challenges Strategies used to address challenges 	<ul style="list-style-type: none"> Use of enhanced consent processes during patient recruitment
Building patient cohorts	<ul style="list-style-type: none"> CDRN/PPRN plans to recruit and retain patients 	<ul style="list-style-type: none"> Key implementation challenges Strategies used to address challenges 	<ul style="list-style-type: none"> Cohort recruitment totals reached Patient survey completed for CDRN cohorts
PCORnet-Level Collaboration			
Facilitating collaboration	<ul style="list-style-type: none"> PCORI/Coordinating Center strategy to facilitate collaboration 	<ul style="list-style-type: none"> Facilitators and barriers to collaboration 	<ul style="list-style-type: none"> Number and type of collaborations Stakeholder perception of collaboration value Cross-network sharing
Engaging Stakeholders			
Engaging patients in governance	<ul style="list-style-type: none"> Patient representation and roles in PCORnet governance and CDRN/PPRN governance 	<ul style="list-style-type: none"> Frequency and type of engagement in PCORnet governance and CDRN/PPRN governance 	<ul style="list-style-type: none"> Patient experience serving in PCORnet governance roles and/or CDRN/PPRN governance roles
Engaging patients in non-governance activities	<ul style="list-style-type: none"> Patient roles in CDRN/PPRN non-governance activities 	<ul style="list-style-type: none"> Frequency and type of engagement 	<ul style="list-style-type: none"> Patient experience participating in CDRN/PPRN non-governance activities
Engaging clinicians	<ul style="list-style-type: none"> PCORI/Coordinating Center plan to engage clinicians CDRN plans to engage clinicians 	<ul style="list-style-type: none"> Frequency and type of engagement 	<ul style="list-style-type: none"> Clinician willingness to support PCORnet research
Engaging health systems	<ul style="list-style-type: none"> PCORI/Coordinating Center plan to engage health systems CDRN plans to engage health systems 	<ul style="list-style-type: none"> Frequency and type of engagement 	<ul style="list-style-type: none"> Health system willingness to support PCORnet research

Network Activity	Implementation Approach	Implementation Process	Implementation Outcomes
Engaging federal and industry partners	<ul style="list-style-type: none"> • PCORI/Coordinating Center plan to engage partners • Partner roles in PCORnet governance 	<ul style="list-style-type: none"> • Frequency and type of engagement 	<ul style="list-style-type: none"> • Partner perceptions of PCORnet's progress • Funder willingness to support PCORnet research

Evaluation Time Frame

The time frame for the external evaluation covered approximately the first 16 months of Phase I. Data collection for the evaluation ceased at the end of June 2015 (after the culmination of the stakeholder interviews). However, in some cases the evaluation team refers to activities that occurred during the last two months of Phase I (through August 2015) to inform the reader of activities that were in progress but that the evaluation team could not fully integrate in its evaluation, given the evaluation's timeline.

Data Collection and Analysis

We used a mixed methods approach for the evaluation that drew primarily on three data sources: stakeholder interviews, quantitative and qualitative analysis of CDRN/PPRN quarterly progress reports, and quantitative analysis of data provided by the Coordinating Center or PCORI. We also used document review and observation of PCORnet meetings (including the PCORnet Steering Committee, Executive Committee, Coordinating Center, individual task forces, and CDRN and PPRN PI meetings) to provide additional context to our findings. These data sources and our data analysis strategy are described in further detail below. In the chapters that follow, we specify the data sources used to assess PCORnet's implementation progress in each activity area.

Data Sources

Stakeholder interviews. A central feature of the external evaluation was semi-structured interviews with a diverse set of stakeholders, including representatives of the CDRNs, PPRNs, Coordinating Center, PCORnet federal and industry partners, and PCORI staff. Stakeholder interviews met three critical needs. First, interviews allowed systematic data collection on dimensions of performance that were not covered or were covered inadequately by existing data sources. Second, stakeholder interviews provided a valuable and efficient method of gaining insight on the facilitators and barriers to success in achieving Phase I goals. Third, the interviews gave the evaluation team the opportunity to learn where perspectives aligned and diverged on the Phase I implementation strategy and stakeholders' perspectives on the success of the network to date.

While participation in the interviews was optional, participation rate among targeted stakeholders was approximately 87 percent, with a total of 170 unique individuals participating in interviews (Table 3.2). Most respondents who declined participation did so because of lack of availability.

We conducted initial interviews with CDRN and PPRN principal investigators (PIs), some of whom invited their project directors to join the interview. These initial interviews included questions that covered most evaluation domains. At the culmination of each interview, we elicited from each network the names of individuals within each CDRN and PPRN who would be best situated to discuss such key topics as patient engagement, data infrastructure, and IRB procedures—three areas that the evaluation team considered critical to the success of PCORnet. We then requested interviews with each of these individuals.

Interviews with Coordinating Center leadership and staff were designed to elicit information on implementation strategies used by the Coordinating Center. We interviewed the leadership of most task forces and, in some cases, project managers as well.

We interviewed all members of the Patient Council using a combination of individual and group interviews, depending on members' availability, to better understand the Patient Council's role, level of involvement in PCORnet activities, and their overall experience serving on the Council.

To understand the perspective of patient representatives who participated in CDRN and PPRN activities, we convened two focus groups of patients. One group comprised patients who were affiliated with CDRNs, while the other group was affiliated with PPRNs. We recruited patients by sampling randomly from among all patient attendees of PCORnet's "Patient Day" retreat that was held during the winter of 2015. The purpose of the Patient Day retreat was to start the process of building a supportive and cohesive PCORnet culture among patients who were members of CDRN/PPRN governance bodies or who were engaged in non-governance activities. In order to operationalize a collaborative patient culture, the retreat was designed to facilitate communication that would lead to a deeper understanding of patients' needs, their vision for PCORnet, and potential participation barriers.⁷⁸ Patients provided feedback on their experience serving on their local governance committee or participating in patient-related activities sponsored by their CDRN or PPRN, which helped the evaluation team better understand how patients were engaged in network governance and operations—one of the novel features of PCORnet.

Interviews with PCORnet federal and industry partners provided the perspectives of infrastructure-building partners and potential end users and funders of PCORnet research on key decisions made during Phase I, their perceptions of the utility of PCORnet at the end of Phase I,

⁷⁸ PCORI, 2015d.

and their outlook for Phase II. We concluded with interviews with PCORI staff, including Program Officers and the senior leadership of PCORI.

Semi-structured discussion guides were developed using information from the Phase I PCORI Funding Announcements, from PCORI and PCORnet documents, from observations of selected meetings, and from the CDRN/PPRN progress reports. Unique interview guides were developed for each stakeholder listed in Table 3.2. Many questions were included in multiple guides to facilitate comparisons of perspectives on assorted topics. Table 3.3 provides a mapping of the content of interviews to specific stakeholder types.

The interview protocol lasted approximately 60–90 minutes, depending on the specific stakeholder. Interviewees were provided with illustrative interview questions prior to the interview. All interviews were conducted in accordance with the requirements of RAND’s Human Subjects Protection Committee and were audio-recorded, professionally transcribed, and periodically audited by the evaluation team to ensure accuracy of the transcription. Interviewees were guaranteed anonymity to encourage candid responses to all questions.

Interviews were held between April and July 2015, with 93 percent of interviews completed by the end of June 2015 (see Table 3.4). All interviews, with the exception of patient focus groups, comprised no more than three respondents each; the majority of interviews were conducted with a single respondent.

Table 3.2. Qualitative Interview Completion Status

Stakeholder Type	Interviewee	Number Contacted	Number Who Did Not Respond or Declined	Number Interviewed
CDRNs	Principal investigators, data leads, patient engagement leads, IRB leads	60	3	57
PPRNs	Principal investigators, data leads, patient engagement leads	70	9	61
Coordinating Center	Co-principal investigators, project management office leadership, task force leadership, task force project managers	22	2	20
Patients	Patient Council, CDRN and PPRN patient representatives	14	3	11
PCORI	PCORI program officers and leadership	9	0	9
Federal partners	FDA, NIH, ONC, CMS, AHRQ, CDC, ASPE	16	8	8
Industry Partners	AHIP, AdvaMed, MDMA, PHRMA, National Pharmaceutical Council	5	1	4
TOTAL		196	26	170

NOTES: AdvaMed = Advanced Medical Technology Association, AHIP = America’s Health Insurance Plans, MDMA = Medical Device Manufacturers Association, NPC = National Pharmaceutical Council, PhRMA = Pharmaceutical Research and Manufacturers of America.

Table 3.3. Content of Qualitative Interviews, by Stakeholder Type

	CDRN				PPRN			Coordinating Center			Patients*	PCORI	Industry Partners	Federal Partners
	PI	Data Lead	Patient Engagement Lead	IRB Lead	PI	Data Lead	Patient Engagement Lead	Co-PIs	PMO	Task Force				
Network Activity														
PCORnet's governance infrastructure														
Decisionmaking	x				x			x	x	x	x	x	x	x
Developing PCORnet policies	x				x			x	x	x	x	x		
Communication and coordination	x				x			x	x	x	x	x	x	x
PCORnet's data infrastructure														
Standardizing data	x	x	x		x	x	x			x		x		x
Developing querying capability	x	x			x	x				x				x
Enhancing data quality/ completeness		x				x				x				x
Protecting patients' privacy		x				x				x				
Collecting patient-generated data		x	x			x	x			x				
Developing biobanking capabilities										x				
PCORnet's research infrastructure														
Streamlining IRB review				x				x		x				
Enhancing informed consent processes				x				x		x				
Building patient cohorts	x	x	x		x	x	x			x				
Building capacity for clinical trials	x	x			x	x		x		x				
Selecting or implementing IRB model				x										
PCORnet-level collaboration														
Facilitating collaboration	x				x			x	x	x	x	x		x
Engaging stakeholders														
Engaging patients in governance	x		x		x		x			x	x			
Engaging patients in non-governance activities	x		x		x		x			x	x			
Engaging clinicians	x				x			x				x		
Engaging health systems	x				x			x				x		
Engaging federal and industry partners								x				x		
PCORnet demonstration projects										x			x	x

* Includes representatives and attendees of the Patient Day retreat.

Table 3.4. Qualitative Interviews to Support the External Evaluation

Stakeholder Type	Interviewee	Number of Interviews			
		April	May	June	July
CDRNs	Principal investigators, data leads, patient engagement leads, IRB leads	11	15	26	5
PPRNs	Principal investigators, data leads, patient engagement leads	16	25	19	1
Coordinating Center	Co-principal Investigators, PMO leadership, task force leadership, task force project managers	7	11	2	0
Patient representatives	Patient Council, CDRN and PPRN patient representatives	0	4	4	3
PCORI	PCORI program officers and leadership	0	0	7	2
Federal partners	AHRQ, ASPE, CDC, CMS, FDA, NIH, ONC	0	0	7	1
Industry partners	AdvaMed, AHIP, MDMA, NPC, PhRMA	0	0	4	0
Total		34 (20%)	55 (32%)	69 (41%)	12 (7%)

CDRN and PPRN progress reports. CDRNs and PPRNs submitted progress reports to PCORI on an approximately quarterly basis as part of PCORI’s contract management activities. For each progress report, CDRNs and PPRNs provided a narrative summary of overall progress and progress toward specific PCORnet goals and milestones, which included both discrete choice responses and open-ended responses. In general, the activities covered by progress reports reflected the network activities described in Table 3.1 above.

PCORI/Coordinating Center quantitative summaries. PCORI and the Coordinating Center provided several quantitative estimates of performance collected as part of operations or internal performance/benchmarking activities. Additional details of these metrics are included in table or figure notes. The external evaluation team did not have any role in the design, collection, or analysis of these quantitative summaries; all summaries were integrated into this report without modification.

Analytic Methods

Stakeholder interviews. Analysis of interview data began toward the end of the interview period. The process involved coding transcripts using *Atlast.ti*, a qualitative data analysis software that marks blocks of text pertinent to specific themes. The coding software was used to identify blocks of text within interview transcripts corresponding to the essential elements of responses to interviews, which allowed the evaluation team to reduce the volume of data and to bring together responses to the same question from multiple respondents to facilitate the identification of themes. Researchers then reviewed the reduced text associated with each

interview question to identify themes that were endorsed by multiple respondents, noted areas of general agreement and areas where opinions were mixed or contradictory, and abstracted quotes that uniquely captured a point of view in participants' own words. Only themes that were identified by multiple respondents were included in the report. Reviewers also took care to appropriately characterize the level of endorsement of a particular theme, using descriptors such as "some," "many," and "most." When perspectives were solicited from different stakeholder groups (e.g., CDRN/PPRN principal investigators and PCORI), we sought to conduct a comparative analysis to identify themes that either converged or diverged between groups.

CDRN and PPRN progress reports. We analyzed a subset of questions from progress reports using quantitative methods to characterize the level of activity or to summarize trends over time in specific areas. We developed abstraction templates specific to each question. For example, to abstract information on collaborations between network partners, we created a grid that identified all of the individual networks with which each CDRN or PPRN collaborated (as reported in progress reports) and also indicated whether or not other networks reported the same collaboration. Two coders used the abstraction templates. Selected questions were abstracted by both coders, who then discussed and reconciled any differences in coding. Differences in the specificity of free text responses between networks and changes in progress report questions over time limited our ability to quantify activities; the summaries included in this report reflect those that the evaluation team found to be most reliable based on the level of code reconciliation required. We analyzed responses to other progress report questions qualitatively for key themes.

In the next five chapters, we summarize the results of RAND's evaluation of PCORnet's Phase I progress within each of five domains: PCORnet's governance infrastructure, PCORnet's data infrastructure, PCORnet's research infrastructure, PCORnet-level collaboration, and stakeholder engagement.

4. Developing and Implementing PCORnet's Governance Infrastructure

One of PCORnet's first tasks was to establish a governance structure for this large and complex research enterprise. Twenty-nine individual networks—most without prior working relationships with one another—came together with the ambitious goal of being able to conduct distributed querying and observational studies within the first 18 months of operation. Developing such a network required the coordinated action of PCORI, organizations making up the Coordinating Center, and the individual networks; therefore, it required the formation of a governance structure to oversee critical elements of PCORnet's development and operations.

Governance is key to the ability of any network to function. This is especially true in the case of a distributed network, which does not share a governance structure by virtue of ownership or contractual affiliation. Governance includes the norms and rules for interacting, making decisions, resolving conflicts, and developing the policies and procedures that are necessary to achieve any sort of collective action. Lack of a clear governing structure (and how it will function) can stymie progress toward other goals.

In order to assess progress in creating a governance structure for PCORnet during Phase I, we asked PCORnet stakeholders questions in three specific domains:

- what they understood to be the *governance structure* for PCORnet and their experience with its functioning during Phase I
- where responsibility for *decisionmaking* lies and their opinion about how effectively decisions had been made in Phase I
- the details of the *policymaking* process and progress toward putting policies in place that will allow PCORnet to function effectively in Phase II.

We found that views varied, based somewhat on the expectations of the individual stakeholder, but did not vary as much by stakeholder type (e.g., CDRN PI, Coordinating Center staff, PCORI staff, etc.) as we expected.

The Initial PCORnet Governance Structure

As context to the discussion, we note that creating a governance structure for PCORnet has been an ongoing effort throughout Phase I. The interviews summarized in the following discussion (with the exception of those with PCORI staff) took place *prior to* the more recent work on development of a formal governance policy document for PCORnet. So, in a sense, the following discussion is a “look back” at an earlier state of the network. We include it, however, because it is too early to tell whether the recently revised governance model will meet the needs and expectations of the network going into Phase II, so lessons learned from the early experience

of Phase I may still be relevant. Additionally, lessons learned may also be useful for the development of future research networks. As noted, the structure developed to govern this network of 29 self-organized smaller networks consisted of a representative Steering Committee, its Executive Committee, and an advisory Patient Council. The Executive Committee was designated by the Steering Committee to develop strategies and processes for PCORnet implementation. Under contract to PCORI but taking direction from the Steering Committee was a Coordinating Center providing technical and logistical support. The task forces established by the Coordinating Center and populated by CDRNs and PPRNs were designed to assist in policy development and sharing of implementation strategies between networks around topics ranging from data privacy to biobanking.

In terms of ultimate authority, according to PCORI, the Steering Committee was intended to “guide members of PCORnet and advise PCORI leadership” but would be “subject to the oversight of PCORI.”⁷⁹

Stakeholder Reactions to the Initial Governance Structure and Processes

Each of these structures was put into place to support PCORnet operations during Phase I. In terms of progress, there were a number of points of general consensus among stakeholders about the initial governance of PCORnet, as described below.

Governance structure was not always clear to participants. We asked stakeholders, “Who runs PCORnet?” A number of respondents said that *they didn’t know* who was running the network—in particular, what entity was making decisions for the network. However, among those who expressed an opinion, two answers predominated: PCORI or the Coordinating Center. Lack of awareness of the roles of governance entities is an indicator of imperfect governance.⁸⁰ Recognizing this challenge, the roles and responsibilities of all PCORnet stakeholders were enumerated and clarified by a work group convened by the Executive Committee in the summer of 2015. These roles and responsibilities were ultimately included in the final governance policy that was reviewed by the Steering Committee at the end of August 2015.

There was a significant minority opinion on governance represented by some of the PPRN PIs. One thought governance was a strength of PCORnet, and others thought that the structure allowed CDRNs and PPRNs to have a voice, although some of those same PIs said that they had been less involved in governance issues than the CDRNs because many of the decisions did not directly affect them.

There was inconsistent coordination between PCORI and the Coordinating Center. There were also “boundary issues” between PCORI and the Coordinating Center, especially early in the implementation of Phase I. One stakeholder put it this way: “There just wasn’t clear

⁷⁹ Fleurence, Curtis, et al., 2014b.

⁸⁰ Schyve, 2009.

role definition,” and another referred to a “blurry line about who’s responsible for what between PCORI staff and Coordinating Center staff.” Later, PCORI specifically restructured the Coordinating Center and moved some of the explicit policymaking functions into its own leadership structure.

Decisions were perceived as “top down.” Critical decisions, like the adoption of the common data model, were perceived by many participants to have been “top-down” decisions. Trust issues developed over the way the common data model decision was handled that lingered throughout the first year of implementation. For some PIs, the common data model decision was a pivotal setback for the initial governance model.

Timelines were not realistic. Many stakeholders felt that the 18-month glide path for initial development and launch of PCORnet was “unrealistic.” The “compressed” timeline, some felt, played into governance problems by making it more likely that PCORI would feel the need to step in and make decisions to move the process along.

Progress and Recommendations for Change During Phase I

Evolving governance structure. The evolution of the governing structure was viewed positively as a sign of adaptability. Some stakeholders reported that the governance structure improved during Phase I, but slowly. In the words of one stakeholder, “it’s getting better [but] on top of an inefficient management structure.” When asked what was needed in a governing structure for Phase II, most respondents did not advocate self-governance for the network. Many stakeholders agreed that some decisionmaking by necessity must be driven by the funder, but they also emphasized the need for a structure that can make decisions for the entire network efficiently and effectively. No one suggested that the Steering Committee or Executive Committee structures should be replaced per se but, rather, that they needed to be modified to function more effectively. One specific suggestion is that the “emerging” Executive Committee needs to be a small-enough group that it can “think and talk and act at a strategic level.” This view is consistent with one of the findings from the CTSA National Evaluation—that “long phone calls involving masses of people” were generally not a good use of time.⁸¹

Election of subgroup leaders. Another change that some PIs advocated is the election of leaders of any governance subgroups, such as task forces or working groups: “if there are any working groups, task forces, or any areas in which we will have to work, [I would like] that the leadership of those groups be elected and not appointed, and elected by the members [of the network] and not appointed.”

Sustainability. Sustainability was on the minds of stakeholders who advocated a change in the governance structure. Some looked forward to a day when PCORnet would be a “spin-off” from PCORI—a separate legal entity—so that governance changes made at the end of Phase I

⁸¹ Frechtling et al., 2012.

were in anticipation not only of the needs for Phase II but also of self-governance at some point in the future. Other PIs felt that there could be a future for CDRNs and PPRNs without PCORI or the Coordinating Center. As expressed by one CDRN PI, “Without PCORI funding, we will continue to exist as CDRNs and most of us have discussed how we can exchange data and work between ourselves.”

Update: Evolution of the Governance Model

There was a general consensus among the principal stakeholders on the need for a new governance model for PCORnet for Phase II. As related by those involved, after a period of “reaching out” and “relationship-building” in the first few months of 2015, a core group (including PCORI staff and some members of the Executive Committee) shared the view that “decisionmaking wasn’t optimal for the kind of network we were trying to build.” Meetings of small groups ensued to “think about how to turn the ship around or re-steer the ship a bit better.” These discussions led to the conclusion that giving more power to the Executive Committee so that it could lead as a transitional board of directors was the appropriate approach.

Under this new approach, “it’s really more of an incubator model where [PCORI] is giving ‘seed money’ for something to grow that would [eventually] become independent of PCORI.” This new view was accepted by PCORI, by members of the Executive Committee, and by the Coordinating Center and began to take hold by the spring of 2015. At the next face-to-face meeting of the Executive Committee (conducted in conjunction with the June Steering Committee meeting), this new emerging role began to take shape.

The new governance policy that was approved by the Steering Committee on the last day of Phase I sets out the PCORnet Council (the successor to the Steering Committee) as the main governing body for PCORnet. The council will have one voting representative from each CDRN/PPRN, one voting representative from the Coordinating Center, and one from PCORI (the PCORnet program director). An elected chair will lead the council, and the PCORnet program director will serve as vice chair.

The Council will have conduct business through the use of several committees:

- an executive committee
- a nominating committee
- a data committee (to oversee the data network, which is managed by the Coordinating Center)
- an engagement committee (to oversee engagement of a range of stakeholders)
- a research committee (to oversee research activities)
- any *ad hoc* committees that the council decides to set up, along with working groups established with council approval
- a PCORnet Advisory Group comprising federal and industry stakeholders (to give input directly to the Executive Committee)

- Chairs of all committees will be chosen by the Steering Committee from a panel of nominations from the nominating committee, and each committee must include patients.

Three major changes are evident (in addition to the fact that the PCORnet Council and its executive committee are playing more of a leadership role than that played by the predecessor Steering Committee): First, the main governing body will be smaller, as it will no longer include the broad range of stakeholders (such as NIH and the other federal agency partners) that were members of the Steering Committee. Second, there are fewer work groups than the task forces they replaced, and the work groups are explicitly under the purview of the committees of the main governing body of PCORnet (and not principally the Coordinating Center). Work groups will be time-limited and tasked with developing or reviewing policies, documents, statements of purpose, and other work products for review and approval by the Executive Committee and then the Council. Third, PCORI and the Coordinating Center will each have only one vote; however, PCORI reserves the right as funder (through August 2018) to “provide leadership and stewardship over PCORnet during this period. Consistent with this role, all PCORnet policies are subject to approval by PCORI.”⁸²

Decisionmaking

The governance structure provided the context in which the members of PCORnet deliberated about and made decisions about PCORnet implementation. To start our inquiry about decisionmaking, we asked stakeholders a broad question: How would you describe the overall process of decisionmaking within PCORnet? We followed by asking if they would characterize the decisionmaking process as either “transparent” or “participatory” and whether they thought CDRNs and PPRNs played a significant role in decisionmaking for PCORnet. What follows are some of the observations made by the stakeholders we interviewed about the processes and outcomes of decisionmaking—including representatives of PCORI, the Coordinating Center, the Patient Council, all task force leaders, and CDRN and PPRN PIs.

The Process of Decisionmaking

Stakeholders varied in their views of whether the decisionmaking process was adequately participatory. Many felt that it did not adequately involve key participants and that it was also not transparent. When asked if CDRNs and PPRNs played a significant role in decisionmaking, one PI characterized PIs as having a “reactive” rather than proactive role. One example related to the numerous task forces that were a part of the initial governance model: “not only was the number excessive, but the leadership was not selected [by us]. And it simply seemed to me that it

⁸² PCORI, 2015c.

was a ‘check box’ to have the CDRNs so-called participation in such activity.” In this instance, the Coordinating Center had gone to great lengths to ensure that all CDRNs were included in each task force; however, some PIs felt that this was counterproductive because they felt “a false sense of participation where most of the decisions were already made.” Other stakeholders, however, complained that the “process” of decisionmaking had been *too* participatory, with “too many cooks” and too much deliberation—resulting in little being achieved.

Update: Evolution of the Approach to Decisionmaking

Some stakeholders report that transparency has improved over time, and PCORI and the Coordinating Center expect that the new governance structure will address concerns that PCORnet is not being governed largely by its members. At the same time, PCORI claims the prerogative as funder to decide for PCORnet what is “mission critical” and to overrule PCORnet governing body decisions if necessary.

Establishing PCORnet-Level Policies

Initial Approach to Policymaking

Initially, policy development for PCORnet was a Coordinating Center function, which was assigned to the Governance and Collaboration Task Force and staffed by an outside collaborating organization (part of the Coordinating Center infrastructure). From the point of view of many stakeholders, it was a “challenging” process from the start. As described by one observer, “one of my takeaways from this effort is that the governance task force, which really sort of owned a big chunk of policy development, really was an idea that essentially failed. And the evidence for that is that at the end of the day, PCORI realized that it had to bring that [policy development] back into PCORI.”

One challenge had to do with responsiveness to the rapid pace of network development. Because the network itself was developing so quickly, it was necessary that the policy development process work efficiently in order to “keep up.” However, the use of an outside organization proved to be problematic. In retrospect, said one stakeholder, “it’s not surprising that they struggled” because the staff were external to PCORnet and, therefore, did not know much about the participating networks or how the networks related to one another. The task force was slow to produce policies and then, at other times, multiple drafts were prematurely circulated before their consistency was confirmed and before legal issues were addressed.

Another problem was that “they [the task force] tried to do it in a participatory way . . .” and without an “operational model” (such as a sample template). This resulted in policies “that ended up being all over the place.” With hindsight, one participant suggested, “what would have made sense would have been to have the very talented people who were on the task force provide their input about what they thought would be important aspects of policy—and then have the PCORI

staff write it. . . . A separate kind of mistake was building a 14-step review process, which, from my perspective, was a very big handicap.” A number of stakeholders agreed that there needed to be a more efficient review process for policies.

When PCORI’s Board of Governors noted the lack of progress in policy development (late fall of 2014), PCORI staff made the decision to take a leadership role in the process of policy development. Even though task force leaders were advised via email of the change, this move was perceived by some stakeholders (who were not directly involved) to have been “abrupt.” Whether or not they agreed that it was abrupt, almost all stakeholders we asked said that they thought it was necessary. Those who disagreed added that a lot of work had gone into the task forces, and they felt that work was overlooked or not used in the subsequent process or thought that the new policymaking process was not reflective of PCORnet values (i.e., not participatory).

Update: Evolution of the Approach to Policymaking

Ultimately, the version of the PCORnet policies that was presented and approved by the Steering Committee was written by a subgroup of the Executive Committee led by the PCORI staff working with a CDRN PI and a PPRN PI. The revised set of draft policies took into account the initial drafts emanating from the task forces. These policies were drafted in about six weeks and circulated for review by individual networks on March 17. Four hundred comments on the various policies were returned to PCORI by the comment deadline in mid-April. Comments were reviewed and a few “big issues” identified that would need to be addressed. These issues came to the Executive Committee, and a small group of committee members considered the comments and developed a final set of draft policies to bring to the Steering Committee for a vote in the summer of 2015.

PCORnet’s Governance: Summary of Key Themes

Governance structure was established rapidly. One of PCORnet’s first tasks was to quickly establish a governance structure for a large and complex research enterprise that had little precedent. Twenty-nine individual networks—most without prior working relationships with one another—came together with an ambitious goal of being able to conduct distributed querying and clinical trials within 18 months. The coordination of efforts by PCORI, the Coordinating Center, and the individual networks of the many infrastructure-building activities necessary to accomplish that goal is an achievement that should not be understated.

PCORnet’s governance has been able to course-correct after recognizing that certain structures or processes were ineffective. The most recent and obvious example was the decision to empower PCORnet’s Executive Committee with greater decisionmaking authority, which helped to allay concerns that the network’s governance structure was not optimally serving the needs of its participants while simultaneously laying the groundwork for a future model that emphasized self-governance. In addition, by reconfiguring the task forces as work

groups under the oversight of the Executive Committee, PCORI took steps to address ongoing governance and operational challenges more effectively and efficiently through smaller groups that have greater accountability and oversight. Finally, PCORI's decision to assume leadership for the development of draft policies as part of an Executive Committee work group was an attempt to better align policies with the evolving governance structure and to simplify a cumbersome process. All of these changes gave PCORnet a perceptible boost in momentum and gave participants hope that many of the future challenges could be resolved quickly and allow the network to engage in research activities.

PCORnet's governance model continues to evolve. We found enthusiasm for the new governance structure but also concern that without strong executive leadership that instills a strong PCORnet identity, participants will be less inclined to work toward PCORnet-level goals and will instead focus on their own network's needs, thereby threatening the viability of the entire enterprise.

5. Developing PCORnet's Data Infrastructure

Development of PCORnet's data infrastructure proceeded in parallel with the formation of the governance structure. PCORnet's data infrastructure rests on two core components: a common data model and a query tool known as PopMedNet. The common data model specifies the meaning of each data element within PCORnet's data network and the required format into which each data element must be transformed so that all data elements are defined consistently across the networks. The transformed data are then available for querying using PopMedNet. As envisioned, the Coordinating Center will initiate queries and transmit them to each PPRN and CDRN. The PPRNs and CDRNs then execute the query code on their local DataMart(s)⁸³ and return the answer to the Coordinating Center, which, in turn, aggregates the responses across the networks. This basic infrastructure, whose development and oversight falls under the purview of the Data Standards and Security Network Infrastructure (DSSNI) Task Force, will support most research activities within PCORnet.

In this section, we present the results of our assessment of PCORnet's experience developing and implementing the common data model and PopMedNet. We also present evaluation results related to activities to enhance and augment PCORnet's data capabilities, including efforts to

- assess and improve data quality and completeness
- develop additional data privacy safeguards
- collect patient-generated data
- develop biorepository capabilities.

CDRNs and PPRNs undertook these additional activities to ensure that the network could support a broad portfolio of patient-centered research with a high level of internal validity while guaranteeing high levels of privacy to the patients whose health information enables the network to exist.

Standardizing Data

Approach to Data Standardization

One of PCORnet's highest priorities for Phase I was for its CDRNs and PPRNs to standardize a core set of data elements. Both CDRNs and PPRNs were expected to standardize a minimum set of data elements by the end of Phase I, including elements in seven required

⁸³ Within PCORnet, a DataMart is a specific data resource that can be uniquely defined and queried using the PCORnet DRN Query Tool. Networks will create their DataMart(s) through an ETL of source data.

domains for CDRNs and three required domains for PPRNs for their target populations (Table 2.4).⁸⁴ CDRNs were permitted to select any subset of patients from their overall target population to achieve that goal.

Selection of the common data model. To standardize data across the network, the Coordinating Center developed and implemented a PCORnet-specific common data model. PCORI and the Coordinating Center used the data model from the Mini-Sentinel project as the foundation for the PCORnet common data model for a number of reasons. First, the Coordinating Center had extensive experience with the Mini-Sentinel common data model,⁸⁵ given its role as the coordinating center of that initiative. Adopting this model allowed the Coordinating Center to launch data infrastructure–building activities very early in Phase I and to leverage many of the analytical tools developed under the Mini-Sentinel initiative. Second, both PCORI and the Coordinating Center viewed the use of claims data in PCORnet as critical to ensuring that the network could conduct CER with complete data (since claims provide a record of a patient’s complete medical care utilization, as compared with EHR data, which may be incomplete if patients seek care from providers outside of the CDRN), and the Mini-Sentinel common data model was developed primarily for use with claims data.

Phased implementation approach. PCORnet used a phased implementation approach to ensure that minimum levels of capability could be developed across all networks quickly. The Coordinating Center released version 1.0 of the common data model on May 30, 2014—only three months after PCORnet’s official launch date of March 1, 2014. It included specifications for demographic and enrollment information; diagnoses and procedures associated with discrete health care encounters; and vital signs, including height, weight, smoking status, and blood pressure. The Coordinating Center released two updates over the course of Phase I—both of which improved the breadth and richness of the data elements included in earlier versions of the common data model. Version 2.0 added specifications to capture outpatient pharmacy dispensing, lab results, patient conditions,⁸⁶ and the Patient-Reported Outcome Common Measures, which are metrics selected by the PROs Task Force (discussed in a later section of the report). Version 3.0 added several new domains, including prescribed medications, PCORnet clinical trial participation information, mortality data (including causes), and such metadata as DataMart refresh dates and use of imputations or date-shifting strategies used by individual networks for specific data elements.

⁸⁴ The common data model tables requiring standardization by CDRNs included demographic, enrollment, encounter, diagnosis, vital, and harvest. For PPRNs, three data tables required standardization: demographic, condition, and harvest (PCORnet, undated[c]).

⁸⁵ The Mini-Sentinel Common Data Model is in its fourth version and contains 11 parent tables in which records are linked by patient ID. It was modeled after the HMO Research Network Virtual Data Warehouse (Mini-Sentinel, 2015).

⁸⁶ These include conditions that may be diagnoses in both health care and non–health care settings and may also include patients’ self-reported clinical conditions.

The Coordinating Center established a new process for implementing updates to the common data model after the release of version 1.0. The expansion of the common data model was subsequently informed by a framework that weighed such considerations as the availability of a data element among network participants, the anticipated burden of standardizing the data, and the potential utility and quality of research using each new data element. The Coordinating Center then solicited feedback from CDRNs and PPRNs on the draft version of the new release, collated the feedback, responded to comments, held webinars to discuss the comments, revised the data model as appropriate, and then released the final version. CDRNs and PPRNs were highly engaged in this process and submitted hundreds of comments on each proposed update.

Implementation Challenges

The data standardization challenges reported in this section draw almost entirely on stakeholder interviews conducted in the last few months of Phase I. Most PPRN and some CDRN respondents reported few major technical challenges standardizing data—citing their experience and informatics expertise as factors that facilitated the process. Coordinating Center representatives also noted that the technical issues were not a major barrier to progress during Phase I. Some respondents indicated that the issues they encountered standardizing data were not specific to the PCORnet common data model but would arise within any data standardization effort. In fact, only one CDRN and one PPRN respondent worked closely with the DSSNI task force and required a high level of technical assistance to implement the PCORnet common data model.⁸⁷

Concerns with decisionmaking. Many respondents criticized the rollout of the common data model during the early part of Phase I. One common complaint echoed the criticism of overall project governance: that decisionmaking in the development of the common data model was top-down, lacking transparency about who was making the decisions and how and why decisions were made, and failing to incorporate participants' feedback into decisions. In particular, some networks had a strong preference for PCORnet to pursue other data models and felt that the final decision was not made with sufficient input from the networks. Ultimately, the Steering Committee as a whole voted to endorse the PCORnet common data model.

Satisfaction with the decisionmaking around the common data model seemed to improve substantially following the shift in common data model expansion process after the initial release of version 1.0. In particular, the Coordinating Center's systematic efforts to solicit and respond to input helped address participants' concerns about their perceived limited role in decisionmaking. In particular, some respondents described the common data model development

⁸⁷ Some CDRN and PPRN respondents worked on data standardization with minimal assistance from the Coordinating Center other than using the Coordinating Center's guidance documents. Many CDRNs and PPRNs were somewhere in the middle: They worked primarily on their own and asked the Coordinating Center only for troubleshooting assistance.

process at the end of Phase I as more collaborative and a process in which the common data model increasingly reflected key use cases, such as those involving both dispensing and prescribing medication—the latter being a data element that CDRNs strongly advocated for being added to version 3.0.

Challenges in model specification. CDRN respondents described the primary technical challenge as transforming data into the common data model specification in a way that ensured that these data provided consistent meanings. CDRNs data leads noted that EHR data and claims data often have different meanings and assumptions; thus, transforming EHR data using a common data model that was initially developed for claims data caused challenges. For example, one CDRN respondent pointed out differences in conventions surrounding dates between the two data sources: EHR data for a surgery are recorded chronologically, whereas claims data are dated based on payment rules, and changes to dates might be made for a variety of reasons. Matching specific events between clinical data and claims data can therefore be very difficult. Similarly, one CDRN described challenges populating the diagnosis field in the common data model because multiple types of diagnoses are available in EHRs, including admitting, discharge, billing, final, and interim operating room diagnoses. The comparability of certain data elements across PCORnet participants will, therefore, depend on the specific mapping decisions made by each network.

Some networks reported considerable work transforming at least some data elements into the common data model. Many mapping steps required lengthy decisionmaking processes to ensure that the transformed data preserved the meaning of the original data. Continuing the example from above, active diagnoses, self-reported diagnoses, rule-out diagnoses, and documentation of a patient’s history of a diagnosis all convey different types of information. Clinicians occasionally conducted manual reviews of the data elements being transformed to ensure that the most appropriate native data elements were used to map to the common data model. Because of variability in data management practices among the institutions within individual CDRNs, one respondent noted that it was challenging to make sure data were being transformed in a consistent way across all sites within the CDRN.

Lack of use cases to facilitate implementation. Many respondents noted that there were few use cases in the early stages of Phase I to guide the development and implementation of the common data model.⁸⁸ This may have slowed implementation because the most appropriate data source for populating a particular data element may depend critically on the specific use case. For example, a hypothetical PCORnet study examining alternative interventions to reduce hospital readmissions might require the diagnosis field to be populated with discharge diagnoses rather than admitting diagnosis codes. Some respondents mentioned that the PRO data elements,

⁸⁸ A use case in this context refers to a sequence of steps that would show how the common data model would be used as part of a research study.

in particular, lacked use cases. For example, many respondents questioned the utility of the Common Measures for PROs; in future PCORnet studies, they may need a more extensive PROMIS-like⁸⁹ instrument to comprehensively assess a patient's health status. Later in Phase I, use cases guided the inclusion of several new elements in the common data model, such as information on prescribed medications.

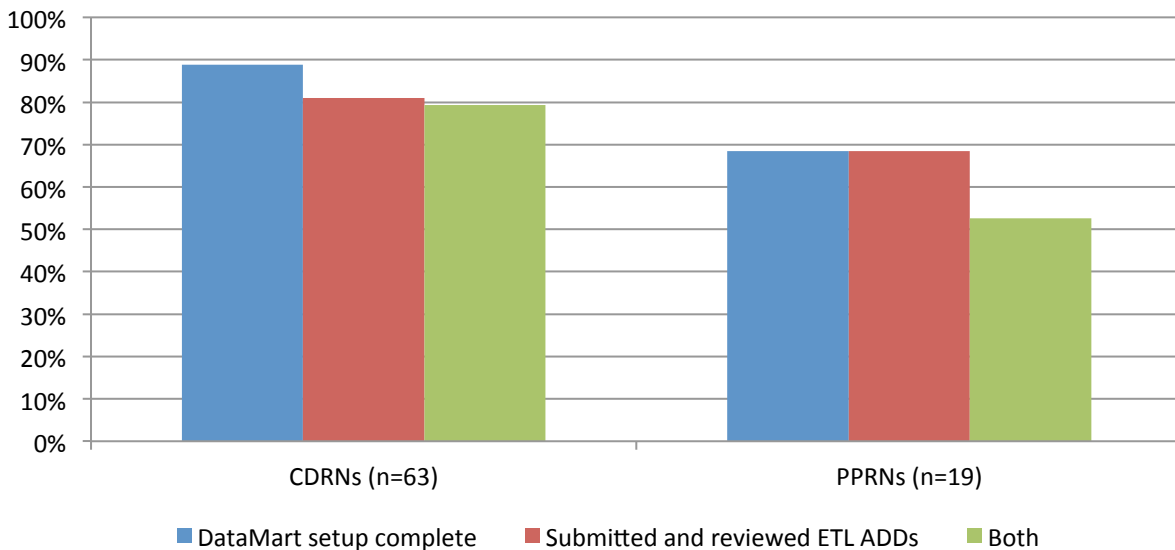
Other challenges. Respondents described assorted other technical, governance, financial, and communication challenges. For example, some respondents were unclear on how to handle data elements with extreme values and what additional data processing steps they should follow, if any. A few CDRN and PPRN respondents noted that resource constraints made it difficult for them to implement the common data model within the allocated budget and timeline. One Coordinating Center respondent pointed out that even though PCORI allowed rebudgeting, the networks struggled to adjust because they had already allocated resources to other priorities and could not easily backtrack on earlier promises made to their CDRN's or PPRN's partners. Finally, several respondents felt that they could have used additional assistance with mapping data to the common data model and that greater opportunities to share lessons learned in the course of transforming data would have been helpful.

Implementation Progress

Progress setting up DataMarts. As of the end of Phase I, 89 percent of CDRN DataMarts and 68 percent of PPRN DataMarts were set up and contained at least some amounts of standardized data. Additionally, 81 percent of the CDRN DataMarts and 68 percent of the PPRN DataMarts submitted and reviewed ETL Annotated Data Dictionaries (ADD), which specify the logic used to transform each data element from the native format used by each CDRN or PPRN into the format specified in the PCORnet common data model (see Figure 5.1).

⁸⁹ The Patient Reported Outcomes Measurement Information System (PROMIS) is a system of patient self-report measures used to determine health status for physical, mental, and social well-being. When used with traditional clinical measures of health, PROMIS data may help clinicians better understand how treatments could affect patients (PROMIS, undated).

Figure 5.1. CDRN and PPRN Progress Setting Up DataMarts



NOTE: These data reflect the 63 CDRN and 19 PPRN DataMarts planned for implementation during Phase I.
SOURCE: DSSNI, 2015.

Progress standardizing data. CDRNs standardized substantial amounts of data using the PCORnet common data model during Phase I. Estimates on the extent of standardization are based on self-reported information in CDRN’s quarterly progress reports. CDRNs collectively standardized data for a total of 72.6 million patients, representing over 3 billion unique patient encounters. Individual CDRNs standardized data on populations ranging from 1 million patients to approximately 24 million patients—indicating that all CDRNs achieved the Phase I requirement of standardizing data on a population of at least 1 million patients. All CDRN respondents reported that they had standardized data for the entire population within their catchment areas, rather than one or more narrowly defined cohorts, which may help to provide a more representative patient population for querying. The level of data standardization completed by CDRNs within the short Phase I timeline represents a significant achievement of the network.

Comparable data on PPRNs were not available, and, as a result, we cannot assess progress on data standardization among PPRNs. PPRNs generally focused on standardizing far fewer domains of the PCORnet common data model than did CDRNs. While many PPRNs hoped to use emerging technology, such as Blue Button (a technical format that allows patients to download their entire medical record), to integrate EHR data into their existing registries and other databases, few were able to do so because the technology did not produce a machine-readable data file. Moreover, one PPRN respondent noted that the expectation for individual patients to obtain a copy of their EHR data and transmit it to the PPRN was unrealistic because few patients would take the time to do so even if the data were available to them. As a result, PPRNs primarily focused on standardizing the common data model’s demographics table.

Perceptions on future progress standardizing data. Respondents from many CDRNs and PPRNs, and even the Coordinating Center, felt that the common data model continues to be handicapped by the relatively small number of data types included in its current version. In particular, several CDRN respondents pointed out that the model was built on a claims data framework and, therefore, did not allow them to take full advantage of their clinically rich EHR data by, for example, using information stored in clinical notes or comment boxes. PPRN respondents focused on the lack of PROs, biosensor data (i.e., data collected from devices that measure biological indicators, such as blood glucose), and disease-specific data that would be useful for the kinds of research they were most interested in conducting. As mentioned previously, some respondents were concerned because the PRO items chosen were selectively drawn from the full instruments and would therefore fail to completely measure their intended constructs.

On the other hand, some respondents acknowledged that there would be gaps in the common data model initially, as the Coordinating Center prioritized data elements that were common to all patients and that would be useful for the greatest number of research questions. Many respondents indicated that a strength of the common data model was that it included many data domains that are critical for clinical research and data elements that were relatively easy to populate for most networks using their existing data. Many respondents said the common data model was analyst-friendly and allowed the reuse of existing analytic programs from the Mini-Sentinel project.

In conclusion, both CDRNs and PPRNs made considerable progress establishing DataMarts, and all CDRNs met their goals of standardized data for over 1 million patients. Moreover, PCORnet developed a transparent and participatory process for updating the common data model to expand the number of data elements that can be queried across the network—a process that has successfully produced two expansions to the common data model. While PPRNs may have made less progress standardizing data, this appears related to the fewer mandatory data domains required to undergo standardization by PPRNs, along with challenges PPRNs faced obtaining EHR data from their patients to enable standardization of a broader set of domains.

Developing PCORnet's Querying Capability

Approach to Developing Querying Capability

A key component of developing research readiness by the end of Phase I was for all CDRNs and PPRNs to develop the capability to respond to electronic queries sent by the Coordinating Center and execute them on their newly established DataMarts. Developing this capability not only requires installing and becoming facile with the query tool itself, PopMedNet, but also developing local governance processes that specify how queries will be received and processed

and how results will be reviewed before they are returned to the Coordinating Center. Both of these components are necessary for fielding a high volume of queries during Phase II.

The Coordinating Center designed a simple query, known as the Initial Basic Query, to assess each CDRN and PPRN's readiness. The query collected counts of unique observations and patients from each data table in the common data model. The initial basic queries began in January 2015 and continued through the end of Phase I and into Phase II.

Implementation Challenges

Information in this section comes primarily from stakeholder interviews and CDRN and PPRN progress reports. In general, most CDRN and PPRN respondents reported few major challenges developing querying capability. Participants were most likely to cite inefficiencies in the process or user interface issues as the main challenges.

Lack of automation. Some PPRNs envisioned a process that was much more automated when Phase I first got under way. Some had even assumed that the DataMart would be part of PopMedNet so that they would be able load their data into it and have queries run automatically. Some thought that the technology should be automated so that it does not rely on copied and pasted queries and results between PopMedNet and their databases.⁹⁰

Work flow and user interface. A few respondents were dissatisfied with the inability to review a query before executing it on their DataMarts. CDRN respondents also reported dissatisfaction with the user interface and suggested that there was room for improvement in usability. For example, one CDRN pointed out that PopMedNet was designed to be used by data analysts, while clinicians found it less user-friendly.

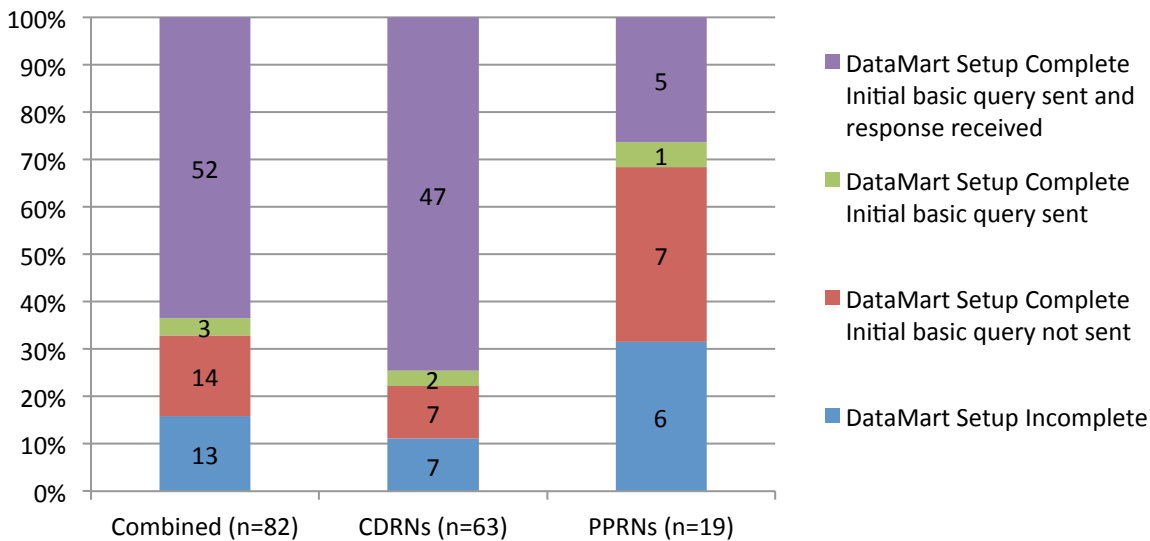
Securing institution buy-in. Some PPRN and Coordinating Center respondents noted that there was a need for extensive education on how distributed queries and the approval mechanisms worked in order to have institutional buy-in, which continues to be a challenge in some networks. This sentiment was echoed in the quarterly progress reports submitted by several networks.

Implementation Progress

Nearly 75 percent of CDRN DataMarts and 26 percent of PPRN DataMarts were able to accept a query from the Coordinating Center using PopMedNet, execute the query, and return the results to the Coordinating Center by the end of Phase I (Figure 5.2). In seven CDRNs and seven PPRNs, the networks had developed the technical capabilities, but the governance processes were not in place to receive the queries.

⁹⁰ Currently queries received through PopMedNet must be copied from PopMedNet and pasted into the local database software (e.g., SAS) in order to be executed, and the results of the query must be copied from the database output and pasted back into PopMedNet.

Figure 5.2. Initial Basic Query Fielding Progress



NOTE: Labels refer to the number of DataMarts that have completed each stage of the Initial Basic Query fielding process.

SOURCE: DSSNI, 2015.

Perceptions of future progress developing querying capability. Many respondents had little feedback on the PCORnet querying approach, mainly because no queries had been run outside of the initial basic query. Several CDRN respondents were frustrated by the lack of querying activity beyond the initial query and felt that, as a result, the capabilities of the query system remained unclear.

Nevertheless, many respondents, especially from CDRNs and the Coordinating Center, mentioned that the PCORnet querying approach had many advantages that should facilitate the continued development of querying capacity. Among its features is the ability to distribute queries and have each one be reviewed by a human and approved by a local governance mechanism, which allows sites to have a clear mechanism to control their data and prevent unintentional disclosures. Many CDRN and PPRN respondents said that the PopMedNet tool was easy to install and simple to use. For example, one CDRN respondent stated that it was “literally like responding to an email.”

In summary, most CDRNs and several PPRNs have successfully implemented the querying infrastructure and have developed the governance processes necessary to respond to queries from the Coordinating Center. Few CDRNs and PPRNs reported implementation challenges. The main challenge facing PPRNs appears to be the initial hurdle of setting up DataMarts. This may reflect the fact that PPRNs varied in their baseline data capabilities as compared with CDRNs.

Ensuring Data Quality

High-quality data collection is critical to any research enterprise. Because EHRs primarily support clinical care rather than clinical research, which requires highly curated datasets, using EHR data for research raises a host of potential data quality issues. Missing data and inconsistent and out-of-range values are common data quality issues that arise when using EHR data. For this reason, Phase I's infrastructure-building activities included assessments of data quality.

Approach to Assessing Data Quality

PCORnet leadership did not require specific approaches for ensuring data quality, as there are few established standards for the evaluation of the quality of EHR data for CER within distributed research networks.⁹¹ All CDRNs and PPRNs were expected to develop and implement a systematic approach to validate data quality during Phase I. Recognizing the importance of data quality for the validity of future research studies, nearly all CDRNs and PPRNs reported ongoing work related to the assessment of data quality. Most were working on making their assessments more systematic, while more advanced networks were actually conducting research projects on the subject.

CDRN approaches. CDRNs, in particular, indicated that data quality was an important and active area of research. Many CDRNs are developing their data quality assessments themselves, and many are also using existing tools, such as those available through OMOP. While many CDRN respondents characterized their processes as still in development, some already had systematic processes in place. CDRNs often apply data quality and completeness assessments both before and after the ETL process to identify errors. Data quality checks used by CDRNs included matching counts between original source data and data transformed into the common data model, using physicians to spot-check unusual values, checking trends over time in data elements, and applying logic checks across data elements (e.g., looking for pregnant males).

PPRN approaches. Some PPRN respondents indicated that data quality checks were done in a mostly ad hoc manner, while others had more systematic processes. Most PPRN respondents indicated that they addressed data quality issues on the data input side, which were typically web-based patient surveys, by using discrete choice questions, limiting ranges of numerical inputs, requiring certain questions to be answered, and conducting logic checks across questions. Some PPRN respondents discussed generating reports of frequencies for ad hoc spot-checking and manual review of outliers by clinicians. Some PPRNs also assessed data quality by using validated surveys that asked the same question in different ways.

PCORnet-wide approaches. The Coordinating Center's plan for assessing data quality used data "characterization" queries based on tools developed as part of the Mini-Sentinel initiative. These queries check to see whether the data adhere to the basic common data model structure

⁹¹ Brown, Kahn, and Toh, 2013; Weiskopf and Weng, 2013.

(e.g., whether the variables are named correctly with allowable values), as well as logic checks of the data elements and numerical checks, such as ratios, trends, and average values. The goal of these queries is to understand the data and the assumptions used to create them.

The DSSNI Task Force used an approach in which a “lead DataMart” within each CDRN is selected by the CDRN for receiving the initial data characterization query. After the Coordinating Center has completed queries for all lead DataMarts, queries will commence for all other DataMarts that are ready to receive queries. As part of this process, the DSSNI Task Force engages the governance entity responsible for each DataMart and discusses the query findings. These discussions are designed to result in concrete steps each site can take to address data quality and to identify issues that might be applicable to all other sites within PCORnet.

Implementation Challenges

Limited CDRN influence over input data. Most CDRN respondents found it challenging to improve data quality because they had no influence over the way the data were captured—particularly data that came from outside of the CDRN (e.g., laboratory result data). As a result, most CDRN respondents focused on managing and keeping consistent their methods of performing data quality checks—which was itself a difficult task. Several CDRN respondents emphasized the importance of local knowledge of workflows and documentation habits in enabling them to understand the nuances of their local data and stated that learning and staying up to date with that knowledge was a challenge. For example, respondents noted that sometimes sites will implement a new EHR system that may lead to data gaps or changes in data formats as a result of the new system. Both CDRNs and PPRNs expressed an interest in having documentation associated with the quality of individual data elements integrated within the PCORnet common data model.

PPRN data quality issues. Unlike CDRNs, PPRNs generally did control data capture. Their challenges included response rates from patients, handling outliers, resolving disagreements with data from different sources, and constructing surveys in ways that produce high-quality responses and limit missing data. Several PPRNs needed to alter phrasing of standard PRO questions to improve their comprehension by patients. Some PPRNs were confident that their data were of high quality and did not report major challenges. Others anticipated many challenges as they expanded data collection.

Patient de-duplication. Several respondents noted that the primary challenge relating to data quality was the issue of patient duplication across institutions within PCORnet. Because patients often visited several hospitals within a few CDRNs, each patient’s records had to be linked to ensure that the same patient would not be counted as multiple patients in the common data model. Several respondents described de-duplicating these records as an ongoing challenge and were exploring methods developed by one CDRN to address this issue. These collaborative efforts are proceeding on an informal basis, although PCORnet leadership brought attention to this CDRN’s work by highlighting it in a “best practices” webinar.

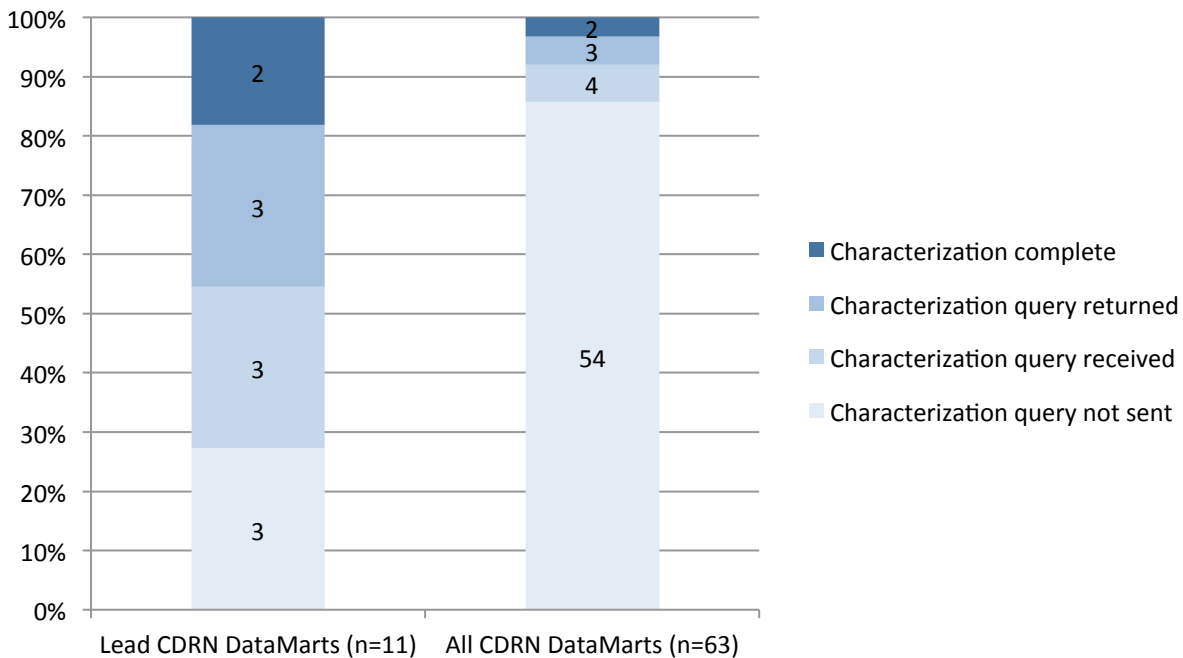
Implementation Progress

Lead CDRN DataMarts have made more progress than have non-lead DataMarts on data characterization (Figure 5.3). As of the end of Phase I, two lead DataMarts have completed the data characterization process, three more have returned their characterization query to the DSSNI Task Force, and three more have received their characterization query.

One respondent noted that the data characterization process may be going more slowly than many CDRNs might hope but also noted that the one-at-a-time process was viewed as necessary to make sure that errors in the queries were identified before sending them to all of the networks. The Coordinating Center expects to have all data characterization queries completed by late fall/early winter 2015. Thus, results from the data characterization queries could not be reported in this report.

In summary, while the quality of PCORnet's data cannot be summarized quantitatively, both CDRNs and PPRNs are actively working on developing systematic approaches to ensure data quality. De-duplicating records appears to be the biggest implementation challenge (which is a potential issue in some but not all networks), followed by keeping up with data documentation protocols. The slow progress in assessing data quality may be due to delays in establishing DataMarts, the one-on-one engagement between the DSSNI Task Force and each DataMart's governance entity to discuss action plans to improve data quality and to identify issues that might be applicable to the rest of the network, and the need to refine the querying process before bringing it to scale. These activities should proceed as quickly as possible to mitigate concerns that funders may have about the quality of PCORnet's data, which might be used by some funders (such as pharmaceutical companies or device manufacturers) to support an application for FDA approval—a high bar. Alternatively, complementary strategies to validate data quality, such as in the context of PCORnet's demonstration projects, should be considered.

Figure 5.3. CDRN Data Characterization Query Progress



NOTE: Labels refer to the number of DataMarts that have completed each stage of the data characterization process.
SOURCE: DSSNI, 2015.

Data Completeness

Patients enrolled in a CDRN might receive care from providers who are unaffiliated with the CDRN, leading to gaps in patient data when using EHR data alone. Supplementing EHR data with claims data is critical to improving the completeness of each CDRN’s data. However, obtaining claims data can be costly, and data from a single payer might cover only a fraction of a CDRN’s patient population. Nevertheless, improving data completeness was a high priority for Phase I to ensure that future studies maintained high degrees of internal validity.

Approach to Ensuring Data Completeness

CDRNs were required to assemble complete, longitudinal data for a cohort of at least 1 million individuals. However, few CDRNs entered Phase I with partnerships that included health plans, and few partners had mechanisms through which claims data might be accessed. As a result, CDRNs were expected to form linkages with additional data partners to improve data completeness over the course of Phase I.

Meanwhile, the Coordinating Center sought an agreement with the Mini-Sentinel data partners (many of whom are large commercial insurers) to supplement CDRNs’ current data with claims. The Coordinating Center also separately engaged representatives from the broader health

insurance industry, as well as CMS, to identify ways in which PCORnet might access commercial and Medicare claims data, respectively, for future research.

Implementation Challenges

All CDRNs reported that their biggest issue in working to ensure data completeness was obtaining claims data from payers and medication-dispensing data from such organizations as SureScripts, the electronic prescribing network. Many CDRN respondents credited PCORnet leadership with identifying data completeness as an issue early in Phase I but were disappointed about the relative degree of responsibility placed on individual CDRNs to work with payers, as opposed to pursuing an entirely PCORnet-wide solution. CDRNs reported several challenges in the course of engaging claims data partners.

Data use agreements. CDRNs cited the restrictiveness of data use agreements as a problem. Even if the CDRN already had claims data in house, existing data use agreements prevented the CDRN from using these data for new studies. Similarly, some state regulations require specific permission for certain sensitive data linkages, causing delays in linking datasets. The issue of the environment in which sensitive linkages would occur (i.e., within the CDRN or by the data partner) was a common issue, with both parties typically having a preference to conduct linkages themselves.

Cost of acquiring and processing claims data. CDRNs reported that the cost of obtaining CMS claims data was extremely high, and some reported the potentially high burden of cleaning and transforming data from partners before it could be used by their CDRN. Moreover, because third-party datasets are static in nature, the data become quickly outdated and the acquisition and processing costs must be borne by CDRNs on an ongoing basis. At least one CDRN reported that state Medicaid agencies were not receptive to CDRN proposals for real-time data linkages.

Gaps in data. CDRNs noted that different payers have varying amounts of data available. For example, one state makes available data on births, deaths, and health care utilization, but not enrollment data from the state's Medicaid program. CDRNs also cited the timeliness of Medicare claims processing as a major issue; the considerable lag in claims processing may pose logistical challenges for clinical trials, which require timely data.

Lack of a value proposition for payers. Several CDRNs reported that without a clear value proposition, payers were much less likely to participate in activities involving data linking.

Implementation Progress

Nearly every CDRN made progress toward engaging claims data partners during Phase I, with varying degrees of success. On their quarterly progress reports, CDRNs reported efforts to link their DataMarts with state all-payer databases (two CDRNs), Medicare claims (typically through initiating data reuse agreements covering Medicare data already available in house) (three CDRNs), Medicaid data, large insurers/pharmacy benefit managers (four CDRNs), and claims aggregators, such as IMS Health (one CDRN). At least one CDRN reported that it had no

plans to work with other payers to obtain claims data and felt that engaging the Mini-Sentinel data partners at the PCORnet level was a much more promising opportunity for acquiring these data.

As of March 2015, when the Coordinating Center systematically assessed the status of data linkages, progress on data linkages was limited. One CDRN reported progress linking Medicaid data that were already in house. Several CDRNs reported that their data use agreements with CMS were still in progress. Other CDRNs reported that they were prioritizing much of this work for Phase II. Of note, four CDRNs reported current or emerging collaborations with HealthCore to facilitate data linking.

Progress was also made at the PCORnet level. An Executive Committee work group focused specifically on data linkages developed a white paper that proposed a model for engaging health plans in creating claims data linkages for patients affiliated with CDRNs and PPRNs. In order to realize the vision of linked data systems, representatives from 18 different organizations associated with Mini-Sentinel and PCORnet began meeting to discuss the governance and technical aspects of data linking associated with several distinct use cases, identify potential challenges implementing the linkage procedures, and provide recommendations for implementation. First convened in January 2015, the group presented the first draft of the white paper to the PCORnet Council in September 2015, with a goal to finalize the paper and all recommendations by early October 2015.

In summary, CDRNs and PPRNs as a whole have actively engaged commercial payers, Medicare, and Medicaid to pursue linkages with claims data. Challenges improving the completeness of their DataMarts include restrictiveness of data use agreements, the cost and data cleaning required of claims data, and the lack of a compelling value proposition for payers to collaborate on data linkages. These efforts conducted by individual networks have yet to bear much fruit. However, PCORnet has provided a potentially useful framework that addresses specific governance and technical challenges that may lead to more extensive collaborations with payers during Phase II.

Developing Data Privacy Standards

PCORnet was launched in a climate of heightened awareness of data privacy threats as a result of several high-profile data leaks. PCORI recognized that data privacy concerns could discourage participation of patients, health systems, and other data partners in the establishment and use of PCORnet. As a result, the development of privacy-enhancing policies was a high priority during Phase I.

Approach to Enhancing Data Privacy

PCORnet's federated infrastructure model, in which data remain behind institutional firewalls rather than pooled centrally, was the primary mechanism in place to guarantee the

security of patients' data. In addition, all queries initiated throughout the network were required to use the minimum data necessary to answer a particular research question. The Data Privacy Task Force was established with the goal of designing minimum data privacy and security standards for the network that would govern data de-identification, data collection, and maintenance. CDRNs and PPRNs were also encouraged to develop additional data privacy policies for their individual networks beyond those that were developed by the task force.

Implementation Challenges

CDRN and PPRN data leads that we interviewed reported few challenges developing and implementing data privacy policies. Their main challenges related to poor communication about networks' requirements and ongoing concerns about the risk of reidentifying patients when responding to PCORnet queries.

Communication and expectations about policies. Respondents described the communication around the expectations on the part of CDRNs and PPRNs to develop data privacy policies as initially being unclear and inconsistent. For example, some CDRNs created a separate database for de-identified data dedicated to querying (and stated their intention of doing so in their Phase I proposals), only later to learn that they were not supposed to do so because some queries needed detailed information on ages, dates of service, and other data elements for the query results to be meaningful. Conflicting guidance from different actors within PCORnet leadership may have contributed to this confusion.

Concerns about reidentification risk. CDRNs appeared to struggle somewhat in their efforts to develop reidentification risk minimization strategies or to certify their data as de-identified in the unlikely event of a data breach. Despite the challenges these networks had when developing these strategies, few CDRNs requested technical assistance from the Coordinating Center, although many did request guidance on other technical issues that were handled through discussions during task force calls or over email by the task force leader.

Implementation Progress

PCORnet-level data privacy policies. The Data Privacy Task Force developed a data privacy policy with guidance from the Coordinating Center and several members of the task force. While the Governance and Collaboration Task Force ultimately approved the data privacy policies, additional work developing standard operating procedures (SOPs) for these policies was deferred until Phase II.

Activity around data privacy within CDRNs and PPRNs has progressed to varying degrees. Some CDRNs reported that they have in place data security and privacy work groups, and at least one PPRN formed a patient-run data privacy work group. Many CDRNs are still engaged in developing their data privacy policies. By contrast, many PPRNs indicated they were waiting for additional policy guidance from the Coordinating Center before beginning to develop their network-specific policies. Several PPRNs that already had data privacy policies in place or in

progress have generally continued to develop them. A few CDRNs were hoping to receive guidelines on minimum data security standards and would have preferred that additional guidance come through the Data Privacy Task Force during the first half of Phase I.

Privacy-enhancing practices. CDRNs and PPRNs pursued a range of activities to strengthen data privacy protections. Most respondents kept patient identifiers in a separate database or table, and several CDRNs and PPRNs used date-shifting⁹² because dates are also considered protected health information. While most respondents understood that date-shifting was not necessary for PCORnet (and was actually discouraged), several used this technique to build trust among partners within their local networks and to reduce the risk of reidentifying patients (particularly among PPRNs whose query results might be more likely to involve small sample sizes). Patients' date of birth was the data element most often cited as being date-shifted. Other respondents cited their query fielding workflows, which include manual review of query results before they are submitted to the Coordinating Center, as another mechanism to protect the privacy of patients whose data were included in PCORnet queries.

PPRN compliance with HIPAA. During Phase I, the Data Privacy Task Force discussed a potential policy that all PPRNs become compliant with the Health Insurance Portability and Accountability Act (HIPAA), as some were not "covered entities" under HIPAA privacy and security regulations.⁹³ Some PPRNs were concerned that HIPAA compliance would prevent them from working with patients in a flexible and adaptive way, particularly with regard to the way they collect patient data. Ultimately, the policy was not adopted.

In summary, across both CDRNs and PPRNs, data privacy has been a high-priority issue. While the structure of the PCORnet distributed research network is, by design, privacy-enhancing, most CDRNs and PPRNs have developed additional privacy protections at the local level. Some networks appear to have delayed work developing highly detailed privacy policies, lacking guidance from PCORnet and the Data Privacy Task Force, which stopped meeting midway through Phase I. Some networks reported challenges developing strategies to minimize the risk of patient reidentification or certifying that their approaches are sound. Identifying solutions to these challenges and additional work developing PCORnet-level data privacy policies (such as minimum standards for all networks) should be a priority for Phase II.

⁹² Date-shifting is altering the date (e.g., a birthday) for purposes of anonymization. For a discussion of date-shifting, see Liu et al., 2009.

⁹³ HIPAA applies only to "covered entities," such as health plans, health care clearinghouses, and health care providers who electronically transmit any health information in connection with transactions for which HHS has adopted standards. PPRNs that do not fall within the definition of a covered entity would not be required to comply with HIPAA, although they might be required to comply with state health privacy laws.

Collecting Patient-Generated Data

One key way in which PCORnet seeks to improve the patient-centeredness of clinical research is through the collection of data on biomarkers, health status, behaviors, and an array of other data elements directly from patients. Generally referred to as “patient-generated data,” these data elements can significantly improve the richness of clinical research. Many PPRNs, in particular, joined PCORnet already having substantial experience collecting and using patient-generated data to engage patients in research and other activities.

Approach to Collecting Patient-Generated Data

The primary strategy for ensuring the availability of patient-generated data to support PCORnet-wide research was the integration of PRO measures in the PCORnet common data model. A set of 21 measures (the PCORnet Common Measures) were included in version 2.0 of the common data model to provide a foundation for the use of PRO data in future PCORnet studies.

CDRNs and PPRNs were also expected to expand the comprehensiveness and completeness of patient-reported data (including an explicit requirement of PPRNs to collect data on at least 80 percent of their population). The most common method of collecting these outcomes was through surveys (which were required for all CDRNs). Additionally, both CDRNs and PPRNs were required to develop processes to enable patients to identify PROs for inclusion into their local DataMarts. Respondents described plans to seek input from patients on the design of surveys, including providing input on the time required to answer questions and the frequency with which the information is solicited.

Implementation Challenges

Most participants agreed that the process for selecting the PCORnet Common Measures was transparent, was participatory, and involved a diverse group of stakeholders. The main complaint participants had with this process, as mentioned previously, was that selected items were taken from existing instruments and may not fully measure the intended construct, and, as a result, some participants questioned the utility of the measures.

Most CDRN and PPRN respondents said that implementing the PRO data elements in the common data model was not technically challenging. However, some mentioned logistical challenges incorporating the data into the EHR and in the common data model. For example, many were concerned that the questions were not relevant to their patient population, and some identified issues harmonizing these items with their existing surveys and had to ask patients to retake surveys.

Many said that there were challenges engaging patients in the collection of large amounts of data for a variety of reasons, including limited incentives for patients to fill out surveys outside of a medical encounter, low health literacy, and the time required to complete surveys. One

CDRN respondent noted that they decided to avoid implementing certain data elements out of concern that clinicians would be obligated to take action in response to the survey responses. For example, questions related to suicide would require clinicians to intervene.

Implementation Progress

Patient-reported outcome data elements collected by PPRNs. We focus on PPRNs' efforts to collect PROs because they faced an explicit Phase I requirements regarding the level of data collection. PPRN respondents indicated that they are currently collecting or planning to collect a broad range of patient data, including measures that fall into five general categories of outcome measures:

- **Conditions, symptoms, and diagnostics:** surveys or online applications designed to track or assess an individual's disease condition, symptoms related to the condition, and associated risk or experiences related to the condition
- **Global health and well-being:** surveys or online applications largely designed to measure an individual's overall health, wellness, or quality of life
- **Health/healthy behaviors:** surveys or online applications designed to track activities and behaviors that contribute to individual health
- **Patient experience of care:** surveys designed to assess patients' experience of their care, their experiences with their providers or care team, or the extent to which they felt engaged in health care decisions
- **Treatment:** surveys or online applications designed to assess the effects or impacts of specific treatments on patient health, physical function, and other components of the treatment experience.

A summary of the types of PROs collected by one or more PPRNs follows in Table 5.1 below. As the table illustrates, most PROs that are currently or will be collected by PPRNs revolve around specific conditions and their tracking or assessment (21 out of 45 identified measures or outcomes). This focus addresses the need for PPRNs to be able to monitor and synthesize information on patients' functioning across a broad set of domains that are relevant for their condition of interest. Following condition or symptom-specific tracking, PPRNs also identified overall health and well-being as an outcome of particular interest (13 out of 45 total identified measures or outcomes). Finally, tracking health or healthy behaviors, patient experience of care, and treatment experiences collectively contributed to about a quarter of all measures identified by PPRNs.

Table 5.1. Patient-Reported Outcomes Collected by One or More PPRNs (Self-Report)

Conditions, Symptoms, and Diagnostics	Global Health and Well-Being	Health/Healthy Behaviors	Patient Experience of Care	Treatment
<ul style="list-style-type: none"> • CCFA PARTNERS: Internet-based registry of patient-reported disease outcomes • COPD Assessment Test (CAT) • Depression and Bipolar Support Alliance Wellness Tracker • GI Buddy Tracker • Modified Medical Research Council Dyspnea Scale (MMRC) • Multidimensional Impact of Cancer Risk Assessment (MICRA) • Muscle Function Module • NeuroTracker • Patient-weighted Disease Progression Instrument • Pediatric Sudden Cardiac Death Risk Assessment • PROMIS—Anxiety; Depression; GI Distress Scale; Pediatric Pain Interference; Satisfaction with Social Roles; Sleep Disturbance; Sleep-Related Impairment • Quick Inventory of Depressive Symptomatology • Seizure Tracker • “What Matters to Me” • World Health Organization World Mental Health Composite International Diagnostic Interview (WHO WMH-CIDI) 	<ul style="list-style-type: none"> • 5-item World Health Organization Well-Being Index (WHO-5) • Health-Related Quality of Life (HRQOL) • Neurological Quality of Life Short Form (Neuro-QoL) • Patient Global Assessment (PGA) • Pediatric Quality of Life Inventory (PedsQL) • PROMIS—Companionship • PROMIS—Emotional Support • PROMIS—Pediatric Global Health Scale (PHG-7) • PROMIS—Satisfaction with Discretionary Social Activities • PROMIS SF v1.1—Global Health • PROMIS-29 Profile v2.0 • RAND 12-Item Short Form Health Survey (SF-12) • RAND 36-Item Short Form Survey (SF-36) 	<ul style="list-style-type: none"> • NHANES Food Frequency Questionnaire (FFQ) • Sleep and activity monitoring app • Sleep-related questionnaire 	<ul style="list-style-type: none"> • Consumer Assessment of Healthcare Providers and Systems (CAHPS) • Decisional Conflict Scale (DCS) • Experience of Care and Health Outcomes Survey (ECHO) • Genetic Counseling Satisfaction Scale (GCSS) 	<ul style="list-style-type: none"> • Functional Assessment of Cancer Therapy—Breast (FACT-B) • Corticosteroid Module • Deflazacort Module

Notably, most PRO measures mentioned by PPRNs consist of existing or validated tools, although some groups have developed their own tools to track patient health and experiences. PPRNs use multiple collection modes, including tablets in clinical settings, paper surveys, and web-based surveys. Most of these data are not transformed using the PCORnet common data model, and several PPRN respondents noted that the common data model contains only a small portion or none of the data they are interested in for their research. Several CDRN respondents noted that they are collecting only what is required by the PCORnet common data model. Some are exploring using data from smartphone apps, fitness trackers, or disease-specific surveys, but they are generally less advanced than PPRNs. Table 5.2 and Table 5.3 summarize the different modalities PPRNs are using to collect PROs.

Table 5.2. PPRN Patient-Reported Outcome Data Collection Strategies

Data Collection Strategy	Number of PPRNs
Patient portals	16
Online surveys	14
Mobile app	4
Interactive voice response	2
Paper-based surveys	4
Other	5

Table 5.3. Number of PPRN Patient-Reported Outcome Data Collection Strategies

Number of Data Collection Strategies	# of PPRNs
5 types of data collection strategies	0
4 types of data collection strategies	3
3 types of data collection strategies	6
2 types of data collection strategies	6
1 type of data collection strategy	3
Total	18

Patient-reported outcome data collection strategies. Patient portals were a particularly common data collection option for PPRNs, and one in which many chose to invest significant time and resources. In many cases, patient portals link directly to the patient’s EHR, and then the EHR data is integrated with data entered by patients through the portal. Other patient portals have less complex functions, such as providing patients with a direct link to surveys or questionnaires, enabling direct data entry by patients, or providing data upload modalities. In all cases, patients have access to their data, and, in some cases, they can easily track and observe

their data as it is compiled and compare their values to those of other patients. Many PPRNs also linked their informed consent process into the patient portal and built dashboards to enable patients to track the types of research activities for which they have provided consent, as well as completed and upcoming surveys. Integration with mobile apps and easy accessibility on smartphones and tablets was commonly considered by many PPRNs, and several focused their efforts on updating patient portals to allow such accessibility.

PPRNs reported several other methods for collecting data:

- Online surveys were also a common method for collecting patient-generated data. Instead of being accessed through the patient portal, online surveys were typically accessed through either the PPRN or partner website or an emailed web link.
- Paper-based surveys were also used by PPRNs, although with less frequency than web-based surveys. Many PPRNs were either developing capabilities with mobile apps or already utilizing mobile apps to collect patient data.
- Apps such as FitBit, Jawbone UP, RunKeeper, and Calorie Counter were commonly mentioned as tools that tracked data on patient steps, fitness, nutrition, sleep patterns, and weight and were capable of being integrated with existing databases or registries.
- A few PPRNs reported the use of interactive voice response⁹⁴ as an option for collecting survey data. Two collaborating PPRNs mentioned the possibility of utilizing an interactive voice response system, but neither had pursued or finalized this option by the time of our interviews.
- Several PPRNs are opting for alternative options to collect patient information, including wearable sensors that continuously collect and upload data, cloud-based storage and transfer options, and in-clinic survey options offered via tablet.

In summary, PPRNs are actively engaged in the collection of (or have plans to collect) substantial amounts of patient-generated data to support research within their networks. PPRNs typically are using multiple instruments and multiple modalities to support collection of PROs. Quantitative summaries of the extent to which PRO data were collected and integrated within CDRN and PPRN DataMarts were not available as of the writing of this report. In addition, an assessment of the process by which PRO measures were selected by CDRNs and PPRNs and the role of patients in their selection was beyond the scope of RAND's evaluation. To address the concerns of multiple participants, future updates to the common data model should consider expansions of the breadth of PROs, including the full set of items from validated scales.

⁹⁴ Kotronoulas et al., 2014.

Developing Biobanking Infrastructure

Biobanks are “systematic collections of samples of human body substances (e.g. organs, tissue, blood, cells etc.) and DNA as carrier of genetic information. Data that contain information on the donor (demographic data, type of disease etc., but also genetic data) are stored, either together with the samples or separately.”⁹⁵ Biobanks provide a critical source of data for an increasingly large body of clinical research. Integrating biospecimen data into a distributed research network like PCORnet has the potential to dramatically expand the opportunities for timely research on cancer, rare diseases, and other conditions.

Approach to Developing Biobanking Capability

Due to many competing priorities and the complexities inherent in developing new biobanking capabilities for networks that had limited experience, PCORnet leadership did not anticipate substantial progress on the development of biobanking capability during Phase I. Among CDRNs and PPRNs that had existing biobanking capabilities at the start of Phase I, it was hoped that these networks would expand the size, scope, and quality of their biobanking operations by pursuing activities in two areas: (1) developing streamlined approaches for obtaining consent for the collection, storage, and reuse of biospecimens and (2) developing systematic approaches for collecting, annotating, and storing biospecimens.

The Biorepository Task Force acted in a consulting capacity for the networks and primarily focused its work on developing strategies to support and enhance individual networks’ biobanking infrastructure, rather than building new infrastructure. The task force created subgroups and generated guidance on such topics as informed consent; biobank sustainability; and aspects of biobank operations including sample preparation, handling, and storage, to assist CDRNs and PPRNs implement their local plans during Phase I.

Implementation Challenges

Competing priorities during Phase I. One of the primary barriers to progress developing biobanking capabilities was the limited amount of attention that networks could devote to biobanking activities during Phase I because of the competing demands of establishing their overall data infrastructure. Task force participants described a mindset within PCORnet that biobanking was simply a lower priority. To some, this lower priority was reflected in the task force’s limited budget, which was only sufficient to complete the task force’s deliverables. The task force was unable to develop practical tools to facilitate the use of biospecimens in actual research studies during Phase I.

⁹⁵ Swiss Academy of Medical Sciences, 2006.

Lack of a biospecimen inventory. While originally planned for Phase I, an inventory of biospecimens across the CDRNs and PPRNs was not undertaken. This information is critical for any study that might seek to leverage these specimens (and related data) because such an inventory would include annotations of the allowable uses for each specimen (per provisions of the informed consent form that governed the original acquisition of the specimens). Of note, the Biorepository Task Force was working on annotation standards to help support observational studies around the time the task force ceased operating.

Challenges related to specimen collection over large regions. A main challenge that CDRNs and PPRNs are facing relates to informed consent and collection of biospecimens from patients who live far from a biobank. This is particularly an issue for rare diseases and patients living outside of the United States. For these patients, a key challenge is obtaining informed consent without an in-person encounter. A related challenge is identifying locations where participants can have the specimens collected and ensuring that the records are made accessible to investigators. One respondent indicated that PPRNs may have more experience with common biobanking systems and that PPRNs are further along with solving these issues than are CDRNs. In addition, PPRNs may be more adept at developing and implementing policies around communicating information pertinent to biospecimens back to patients.

Long-term sustainability. The task force recognized that a chief concern among CDRNs and PPRNs would be sustainability of biobank operations and, as a result, generated policy guidance specifically on network sustainability. Over the course of Phase I, task force discussions often revolved around the topic of sustainability, suggesting that this area will remain a concern for both CDRNs and PPRNs in Phase II and beyond.

Implementation Progress

Despite the limited focus on biobanking, many CDRNs and PPRNs continued to develop their existing biobanks during Phase I. While task force members were not aware of biobanks that were newly launched during Phase I, the task force did receive many queries about starting biobanks, suggesting that it was an activity that networks were interested in pursuing.

CDRN progress. Most CDRNs indicated that they are actively engaged in biobanking activities, although the level of effort differs across networks. Several CDRNs are still in the early planning phases, and activities are limited to drafting specific biobanking strategies and policies. For example, one CDRN reported that it was compiling biobank consent and protocol forms and formed a biorepository committee to discuss plans and next steps. However, about half of the CDRNs reported that they are in advanced stages of biobank planning and implementation. One CDRN indicated that its network's biorepository work group has met several times and is establishing an informatics framework that will allow it to integrate specimen data into its research database while making its specimens searchable by the research community.

PPRN progress. The majority of PPRNs are also pursuing biobanking activities (even though they are not obligated by their PCORnet contracts to do so). Many PPRNs are still in the early planning phases for establishing their biobanks. For example, one PPRN reported that it has been meeting and exploring potential relationships with biobanking companies, and another PPRN is negotiating with NIH about possible funding opportunities for biobanking activities. Some PPRNs are in more advanced stages of implementation, and a few are actively collecting biospecimens and genetic test results. On the other hand, many PPRNs elected not to focus on biobanking at all during Phase I.

In summary, although the level of biospecimen collection likely continued at a steady pace among network that had established biobanks during Phase I, many CDRNs and PPRNs developed committees and began planning activities to support the development of these capabilities in Phase II. Both CDRNs and PPRNs appear to be concerned with issues of sustainability and improving the logistics of data collection. An inventory of biospecimen data within PCORnet and its available uses appears to be a high priority for PCORnet in Phase II, as well as the development of SOPs to facilitate the use of biospecimens in actual research.

Data Infrastructure: Summary of Key Themes

Progress on data standardization. In the span of 18 months, PCORnet released the common data model, followed by two expansions. By the end of Phase I, all CDRNs had developed the capacity to access nearly 200 standardized data elements measured on populations exceeding 1 million individuals. Moreover, the DSSNI Task Force has developed both a framework for prioritizing data elements for addition to the common data model and a structured approach for soliciting and responding to stakeholder feedback for each proposed expansion. CDRNs and PPRNs have shown enthusiasm for this participatory model and have acknowledged that their contributions are reflected in the content of common data model expansions. The common data model rests on a strong foundation that should continue to support PCORnet research in the coming years.

Progress on developing querying infrastructure. CDRNs and, to a lesser extent, PPRNs have made considerable progress setting up their DataMarts and responding to test queries. Over 80 percent of CDRNs and 53 percent of PPRNs have set up DataMarts and submitted ETL data dictionaries establishing the rules for handling transformation of each site's native data into the PCORnet common data model format. More than 60 percent of DataMarts (75 percent of CDRN DataMarts and 26 percent of PPRN DataMarts) were able to accept test queries, execute them, and return the results. These data suggest that a sizable number of CDRNs and PPRNs have made progress implementing PCORnet's querying infrastructure. However, the fact that not all CDRNs have completed test queries at the end of 18 months raises some concern that some CDRNs may be struggling to implement the querying infrastructure or are having challenges standardizing data.

Data quality. Another potential concern is the quality of PCORnet data. To date, the DSSNI Task Force has conducted systematic assessments of the quality of the data for only two CDRN DataMarts. As a result, RAND has insufficient information to characterize data quality. Some participants have expressed frustration with the pace of DSSNI's assessments; however, the Coordinating Center has deliberately employed a rolling strategy for these assessments to ensure that the process can be optimized and lessons learned about data quality problems can be disseminated before engaging all sites in data characterization queries. These activities should proceed as quickly as possible to mitigate concerns that funders may have about the quality of PCORnet's data, which might be used by some funders (such as pharmaceutical companies or device manufacturers) to support an application for FDA approval—a high bar. Alternatively, complementary strategies to validate data quality, such as in the context of PCORnet's demonstration projects, should be considered.

Ensuring complete data. One of the biggest threats to PCORnet's research enterprise remains the ongoing challenge of obtaining complete, longitudinal patient data. Estimates on data completeness were not available to RAND, but stakeholders widely acknowledge that this could be a threat to future research. Despite efforts by many CDRNs and PPRNs to obtain reuse agreements for data already in house or to acquire new claims data, many described considerable financial or administrative burdens of doing so and payers who were reluctant to engage in partnerships. Many networks expressed a strong preference for a PCORnet-level solution, as negotiating with individual plans was seen as inefficient and costly. However, efforts to engage payers at the PCORnet level have yet to produce an agreement with a national payer. A more aggressive outreach strategy may be needed to quickly gain commitments from payers. Otherwise, gaps in data completeness could significantly constrain the type of research that PCORnet can undertake in its next phase.

Ensuring data privacy. Most CDRNs and PPRNs have developed privacy policies locally, but some networks have delayed work on highly detailed privacy policies, lacking guidance from PCORnet and the Data Privacy Task Force. Some networks have faced difficulties developing strategies to minimize the risk of patient reidentification or certifying that their approaches are sound. One network has pioneered new techniques to link patient records using encryption-based technologies, which several networks are now seeking to implement. Developing PCORnet-level data privacy policies and SOPs remains a priority for Phase II.

Collecting patient-generated data. While quantitative summaries of the level of PRO data collection by CDRNs and PPRNs were not available as of the writing of this report, PPRNs were actively engaged in the collection of (or have plans to collect) large amounts of patient-generated data to support research within their networks.

Developing biobanking capability. Developing biobanking capabilities understandably received low priority during PCORnet's first 18 months. Only a limited number of sites began Phase I with meaningful biobanking capability, and work on the common data model received top priority. However, PCORnet should consider accelerating the development of this capacity as

it heads into its next phase to help to distinguish it from other networks—particularly as personalized medicine initiatives receive increasing attention at the national level. As a first step, developing a biorepository inventory could help strengthen the value proposition for potential funders. As a network, PCORnet will have to develop standards and protocols for the use of biospecimens in future PCORnet studies, and many networks with existing capacity are concerned about the long-term sustainability of these activities. Thus, while there appears to be a lot of interest in this area among participants, considerable work remains ahead to develop biobanking capacity across PCORnet.

Participants and potential funders share concerns about the ability of the PCORnet common data model to support a wide range of research, given its current limited scope. For instance, while the common data model is helping to support recruitment for the ADAPTABLE trial, the common data model alone is unable to cover all of the trial’s eligibility criteria, which may lead to less efficient recruitment. Additional demonstration projects will be needed to test the suitability of the common data model for both recruitment and measuring patient outcomes. Expanding the common data model in a way that accounts for differences in priorities among participants will be an ongoing challenge. However, tensions could be diffused if the common data model can be expanded rapidly.

Research readiness also requires CDRNs to have the capacity to field potentially hundreds of queries per year while meeting PCORnet standards for turnaround times and also ensuring that query results do not risk identifying patients. It remains unclear to what extent CDRNs have the governance processes or manpower to field such a volume of queries. The Initial Basic Queries provide a limited indication of whether CDRNs and PPRNs will be able to accommodate this level of querying. Moreover, PCORnet will need to consider testing more complex queries that access multiple tables of the common data model concurrently and may pose additional data quality challenges.

6. Developing PCORnet's Research Infrastructure

In parallel with building the data infrastructure, developing the PCORnet research infrastructure was one of Phase I's central activities. The RAND team evaluated progress in three domains:

- **Implementing multi-site IRB processes.** Institutional review of clinical research ensures that patients are adequately protected against a variety of risks associated with research, but it also adds administrative costs and may lead to delays in study initiation. The use of centralized multi-site review processes was viewed by PCORnet as a key strategy for improving the speed and lowering the cost of research.
- **Developing patient-centered consent processes.** Clinical researchers have identified numerous limitations of current consent processes. As PCORnet strives to implement a patient-centered model of research, improving the consent process was considered an area in which CDRNs and PPRNs might innovate as PCORnet's infrastructure was being established.
- **Enrolling patients into cohorts.** A core requirement for Phase I was to enroll patients into networks in order to identify cohorts of patients who are "ready" to participate in clinical research. The availability of large groups of patients who are willing to enroll in research studies could help jump-start research on multiple projects with little lead time required.

Implementing Multi-Site IRB Review Processes

Approach to Implementing Multi-Site IRB Review Processes (CDRNs)

CDRNs were required to develop and implement a multi-site IRB review process within their networks to expedite the review of proposed studies for research within their CDRN and, eventually, for PCORnet-wide research. Each CDRN was given the flexibility to select a model that was best suited for its network. By the end of Phase I, three distinct models had emerged: (1) IRBShare⁹⁶ (two CDRNs), (2) reliance agreements⁹⁷ (six CDRNs), and (3) central IRBs (three

⁹⁶ IRBShare is a collaborative IRB review model in which a designated lead site of a multi-site trial conducts the full review of a study protocol and uploads the resulting documentation to a secure web portal for use by other participating sites. IRBs associated with these sites can then access this information, accept or reject the decision of the lead IRB, and conduct additional reviews for issues of relevance to the site.

⁹⁷ A reliance agreement is a formal, written document that provides a mechanism for an institution engaged in research to delegate IRB review to an independent IRB or an IRB of another institution" (NIAID, 2015).

CDRNs) (see Table 6.1). IRBShare and reliance models are relatively recent innovations that had been adopted by a fairly limited number of institutions nationwide prior to the launch of PCORnet.

Table 6.1. CDRN Use of Streamlined IRB Models (Self-Report)

Streamlined IRB Model	Number of CDRNs
IRBShare	2*
Reliance agreements	6
Central IRB	3

* Both used reliance agreements for most of Phase I but are moving to IRBShare for Phase II.

IRBShare. Both CDRNs that selected IRBShare began Phase I using a reliance model, with each CDRN’s lead site serving as the IRB of record. In one case, the decision to migrate to IRBShare was based primarily on Phase II considerations (the CDRN’s new partners were existing members of IRBShare), whereas in the other case, the model emerged over time as the most satisfying option among the CDRN’s existing partners.

Reliance agreements. Several CDRNs had extensive experience developing and using reliance agreements as members of regional research networks prior to joining PCORnet. Two CDRNs developed reliance agreement templates through the Clinical and Translational Science Award (CTSA) program, while another is currently engaged in developing a national reliance agreement for the CTSA program.⁹⁸ Each of these CDRNs leveraged its existing reliance agreements to quickly initiate new agreements among their PCORnet partners.

Central IRB. Three CDRNs use central IRBs for PCORnet research. Two used existing regional or nationally recognized central IRBs that have been operating for over 15 years each, whereas the third created a new IRB whose sole purpose was to conduct reviews of PCORnet research protocols.

Additionally, under the reliance model, institutions participating in multi-site studies develop a network and agree to rely on a single IRB of record that is responsible for reviewing, approving, and monitoring the study (NCATS, 2015). Institutions may use different descriptive terminologies when discussing reliance agreements, including “cooperative agreements,” “IRB authorization agreements (IAA),” or “memorandums of understanding (MOUs)” (NIAID, 2015). A “ceded” IRB review is similar to a reliance agreement in that the ceding IRB is an IRB that hands over authority and oversight responsibilities to a lead IRB (HMORN, 2008).

⁹⁸ The Clinical and Translational Science Award program is an NIH-funded program that seeks to accelerate the translation of research into new drugs, diagnostics, and medical devices through engagement and innovative approaches to research across the spectrum of translational research activities.

Implementation Challenges

CDRNs reported a number of challenges in the course of implementing these models, including negotiating institutional- or state-specific policies into agreements and reluctance on the part of one or two partners to cede IRB review to a lead or central IRB.

Challenges specific to IRBShare. CDRNs that used IRBShare noted that there was a learning curve that sites unfamiliar with the process had to overcome. In particular, limiting the focus of the review by ceding sites to “local context” considerations only (such as pharmacy, billing, subject injury, etc.) is a significant change for local IRBs that are accustomed to reviewing entire protocols. Other CDRNs described legal issues, including one CDRN, whose partner invested nearly two years getting its regulatory and legal teams to support the use of IRBShare before signing the master agreement. A CDRN that declined to use IRBShare noted that its legal counsel refused to accept the IRBShare master agreement because of its indemnification and insurance clauses. In particular, the CDRN’s lead site noted that, as a state institution, it could not agree to most indemnification clauses. Finally, one CDRN cited that its slow progress was due to the fact that IRBShare’s master agreement was in the process of being updated to address language on liability insurance coverage, which had prevented many other institutions from signing the agreement.

Challenges specific to reliance agreements. Respondents noted that the main barrier to signing reliance agreements was specific language that individual institutions had to negotiate to comply with state laws. In addition, policies governing reportable events were also cited as being challenges with all reliance agreements, such as noncompliance, unanticipated problems, and adverse event reporting. As a result, one CDRN respondent made the reliance agreements as stripped down as possible and included all of these details in the SOPs, which are separate documents. The CDRN noted that most institutions allow IRB directors, rather than their legal department, to approve SOPs on behalf of the institution, and this greatly facilitates the process.

Challenges implementing central IRB models. CDRNs reported few challenges with their central IRBs. According to one respondent, the only concern was whether or not the IRB fully understood the local network, patient population, and the nature of the research that the CDRN was pursuing. This CDRN saw value in gaining the perspective of a second IRB to validate the conclusions of the central IRB and to determine whether the initial review was sufficiently rigorous.

Lack of a PCORnet forum to address IRB issues. While nearly all CDRNs reported that they did not require technical assistance to help them implement their streamlined IRB model, they did have an interest in exchanging information on best practices. One CDRN noted that, after the Ethics and Regulatory Task Force stopped meeting, there were no other forums in which IRB issues could be discussed in depth across PCORnet until the Executive Committee’s IRB work group was established during the summer of 2015 (discussed below). Several stakeholders felt that the task force did not effectively promote communication around IRB-related challenges

even when it was active. Another CDRN felt that the series of 11 white papers on various ethics and regulatory topics spearheaded by the task force was a necessary and valuable contribution and was an important first step for PCORnet, but this CDRN would have preferred that the task force take up some of the more “practical issues” at the same time.

Challenges associated with PCORnet demonstration projects. The Coordinating Center selected IRBShare as the review model for the ADAPTABLE trial.⁹⁹ As a result, individual sites that wanted to participate in the trial were required to use IRBShare, rather than the centralized review process that CDRNs had been developing since the beginning of Phase I. This choice frustrated several CDRNs. One felt that this decision would lead to a lost opportunity to test the performance of the three basic review models in place among the CDRNs. For at least one CDRN that is using a central IRB, the implications of the decision to use IRBShare were still being discussed as of the writing of this report, and there remains the possibility that individual networks will have to conduct reviews locally if the CDRN’s central IRB refuses to defer using the IRBShare system. In another case, the CDRN’s central IRB will review the protocol independently of IRBShare because it was determined that it would take too long for individual sites to become members of IRBShare.

Implementation Facilitators

Prior experience was, by far, the most important factor associated with successful implementation of streamlined IRB review models during Phase I. Several CDRNs also described the commitment of experienced IRB staff, the ability of existing templates, and flexibility on the part of participants as key facilitators.

Experience and dedication. As described above, most networks selected an IRB model with which they had some experience that they could, therefore, feasibly implement within Phase I’s short timeline. Several networks cited the involvement of senior IRB officials/administrators who are both experienced and highly motivated as a main reason why CDRNs were able to gain buy-in from their PCORnet partners to adopt the model. One network described its work on reliance agreements as a “labor of love” that drives its staff to get to know its collaborators closely and also helps to build trust that all parties will work to make the reliance agreements meaningful and acceptable to all partners. For one CDRN with extensive experience with reliance agreements, face-to-face meetings held multiple times a year were seen as a critical factor to help reinforce the agreements once they were in routine use.

The availability of templates and examples of successful execution of a model (particularly reliance agreements) were seen as helpful in making progress during Phase I. Some CDRNs began Phase I with examples of reliance agreements that their new partners could review that allowed them to make progress quickly. To encourage participants to sign its reliance agreement,

⁹⁹ See Chapter 3, “Evaluation Methodology,” for a description of the ADAPTABLE trial.

one CDRN developed a “single IRB Review Board policy” that required all existing and all future partners of the CDRN to endorse the policy of utilizing a single IRB model, although reliance on any IRB for any individual study remained voluntary.

A few CDRNs mentioned the willingness to embrace a cultural change in the way IRB reviews are conducted as a contributor to successful implementation of these new models. One CDRN noted that the process of implementing its central IRB has enabled all its partnering institutions to rethink their own positions on various issues, including whether institutional policies address core values or whether they are grounded in custom and need to be reconsidered. This CDRN noted that meeting on a regular basis has helped the CDRN’s partners with both conservative and liberal approaches converge toward a middle ground. Another CDRN similarly reported wide variability in institutional culture across its sites with respect to IRB review, and, as a result, it added community stakeholders from each site to try to create a new culture that facilitates agreement on IRB review processes.

Progress Implementing Streamlined IRB Models

In this section, we report CDRNs’ progress implementing multi-site IRB protocols, based on discussions with CDRN IRB leaders as of the end of June 2015.¹⁰⁰

Approval for PCORnet queries. Most CDRNs had few problems gaining IRB approval for their protocol for fielding and responding to PCORnet queries. Some CDRNs noted that their sites already had approvals in place because they had existing querying systems (e.g., i2b2). Most CDRNs noted that once IRB approval was in place for the PCORnet data infrastructure, querying protocols did not require approval because they were considered preparatory to research, but not research.

IRBShare. The IRBShare sites have signed the master agreements and established reliance agreements for sites that elected not to become members of IRBShare. One CDRN has used the process for non-PCORnet research successfully, while the other CDRN was still in the process of developing SOPs and has yet to submit a study protocol through the system.

Reliance agreements. Among the CDRNs using a reliance model, all partnering sites within five of the six CDRNs have signed the reliance agreements. In the sixth CDRN, a mixed approach was ultimately adopted in which most sites (all of which were affiliated with the same

¹⁰⁰ The evaluation team did not interview PPRN IRB leads. Through examination of quarterly progress reports, we note that a majority of PPRNs gained IRB approval for Phase I activities through academic institutions at which they were based or with which they were affiliated. A small number of PPRNs opted to either include or exclusively pursue a multi-site IRB review model, including a master reliance agreement and a central IRB. A majority of PPRNs appeared to have several sites at different stages in the IRB review process but did not express that this created any significant barriers to completing their Phase I deliverables. By January 2015, a majority of PPRNs had either submitted protocols covering their Phase I activities to their IRBs or had already received IRB approval. Nearly half of all PPRNs submitted protocols for research studies and had them approved by their IRBs by approximately one year into Phase I.

university) agreed to use a reliance model, whereas all other sites (all of which were affiliated with a single health system) decided to use their own health system's central IRB. In most cases, the agreements were signed relatively quickly, and one CDRN was able to sign all agreements and establish all SOPs over a period of only eight months—which the CDRN reported as being uncommonly fast.

Central IRB. All three CDRNs that used a central IRB have made considerable progress implementing the model during Phase I. In two CDRNs, protocols for the three observational cohorts and for the data network as a whole were approved. In the third CDRN, half of the sites have agreed to rely on the central IRB (all of these sites already had agreements in place), while half of the sites had yet to sign the agreement at the time of our interviews. A representative of this CDRN noted that gaining consensus on the terms of the reliance agreements took somewhat longer than expected because of the large number of academic institutions whose individual review processes were quite extensive.

These three CDRNs reported mostly positive experiences with their central IRBs. A single site within one CDRN agreed to the model but refused to cede to the central IRB for any study that requires direct patient consent. A second CDRN had difficulty getting a Veterans Affairs Health System site to agree to the central IRB, which was not unanticipated. Aside from these few cases, these CDRNs have experienced few challenges. In fact, one CDRN reported that across its nearly ten institutional partners, few wanted major changes to the Central IRB agreement. In addition, one CDRN reported being pleased with the IRB's quick turnaround time, in which one IRB determination was completed in less than ten business days.

PCORnet-wide IRB review processes. The Executive Committee established a work group in June 2015, to address IRB review protocols for PCORnet-wide studies. This work group was convened to investigate potential models for all participating networks and to develop a plan for its implementation during Phase II. By August 2015, the work group had held webinars for the PCORnet community about different IRB review models (specifically IRBRely, IRBShare, and IRBChoice) and fostered discussion about the ways that they could be adapted or integrated into PCORnet. As Phase I drew to a close, the work group was well on its way to achieving the goal of developing and finalizing an IRB review model by the end of the calendar year.

In summary, CDRNs have made considerable progress implementing streamlined IRB models during Phase I. Some CDRNs had delays or difficulty obtaining buy-in by all sites within their network because of clauses that raised legal concerns, or because partnering sites had preferred to use their existing models (which included central IRBs in several cases). CDRNs expressed interest in more opportunities to discuss these and other “practical” implementation challenges with other networks. In general, CDRNs' IRB review processes appeared to work fairly well for Phase I's “data only” protocols, and CDRNs' substantial experience with each model helped to speed implementation. Some culture changes may be under way as individual institutions question their former review processes. The real test may lie in the future, when

PCORnet begins conducting greater numbers of interventional studies. Institutions will likely vary in their tolerance for ceding reviews to other institutions, depending on the specifics of each trial. PCORnet’s decision to recommend a common IRB streamlining model may also pose challenges and possibly resistance among CDRNs that have adopted alternative models during Phase I.

Implementing Patient-Centered Consent Processes

Informed Consent Approaches

While many CDRNs and PPRNs sought to improve informed consent processes during Phase I, competing priorities—in particular, implementing multi-site IRB review processes—often limited the amount of time and resources that networks could devote to the issue. Similarly, several CDRNs noted that enrolling patients into the three required cohorts entailed waivers of written informed consent and, thus, many CDRNs focused more on recruitment processes than informed consent per se. CDRNs and PPRNs that worked to enhance informed consent often sought new ways to minimize the burden on clinic staff of administering consent in person; several pursued electronic consent approaches. CDRNs were also mindful of the need to support participants’ understanding of consent documents, with one CDRN implementing a series of questions within its electronic consent process that the potential research participant must answer correctly to be included in the study. Only one CDRN decided not to focus on consent issues during Phase I.

Implementation Challenges

Participants noted a number of challenges developing new consent processes, including gaining consensus around interpretations of federal requirements for consent, differences in policies across sites, and lack of guidance at the PCORnet level.

Interpreting common rule requirements. Several respondents cited the complexities of developing innovative consent approaches because of the federal regulatory requirements around the required elements of consent. For example, one CDRN noted that some institutions were very conservative in their interpretation of the regulations, and so all partners needed a prolonged period to negotiate and reach consensus on the language of a common consent template. Moreover, PCORnet leadership noted that there remained “huge issues that were completely unresolved at the [federal] level about the Common Rule and how it was going to be applied that are still playing out today, so you could’ve spent a lot of time dealing with details of informed consent only to have it completely changed or reversed.” As an example, one CDRN noted that the requirements for informed consent in the context of cluster randomized trials of minimal risk studies remain hotly contested.

Variation in local approaches. Several CDRNs noted differences in preferences for consent processes among the members of their individual CDRNs. This included disagreement even for such basic questions as whether or not to waive consent. One CDRN explored disagreement systematically by distributing a small number of hypothetical scenarios to the CDRN’s regulatory work group to determine when consent would be required or waived and found that there was little uniformity of opinion among the CDRN’s sites. Differences in perceptions of the literacy level of informed consent forms caused some tension among the partners within at least one other CDRN. Finally, one CDRN noted that policies around approaching patients for recruitment into studies varied widely across institutions.

Lack of guidance at the PCORnet level. Some CDRNs expressed the desire for more guidance from PCORnet leadership so that they could align informed consent strategies locally, although this was viewed less as a problem than as a missed opportunity. For example, according to one CDRN, the requirement to use a common consent approach for the ADAPTABLE trial provided an opportunity to develop a consensus approach that might have provided direction to the network as a whole. Similarly, one network expressed preference for an informed consent “policy” from PCORnet that the CDRNs could use as the basis for local implementation. Another CDRN thought that there was a unique opportunity for consensus-building around the interpretation of regulations.

CDRN Progress on Informed Consent

Developing informed consent templates. Several CDRNs are in the process of developing informed consent templates for use by all sites within their CDRN, which may or may not be administered electronically. These forms typically contain sections that allow some flexibility to meet the unique needs of each study or to allow individual sites to add their own language to address policies that vary by site, such as policies on reimbursement and liability.

Exploring and developing e-consent options. Several CDRNs are laying the groundwork to shift to an e-consent strategy. Among the CDRNs that have made notable progress implementing e-consent processes during Phase I, two stand out for having substantially overhauled their approach. One CDRN sought to develop an entirely new consent process using its Phase I funding (a longstanding goal of theirs) that would allow them to embed pictures and videos, as well as helpful links to additional information, so that patients can be more meaningfully engaged in their decision to participate in research. Another CDRN developed an enrollment app that patients can access through tablets when seeking care in the CDRN’s clinics.

Patients’ reactions to these new tools have been overwhelmingly positive. According to the two CDRNs, patients value the added features they offer and are reassured by the fact that they can get help whenever they like.

PPRN Progress on Informed Consent

Electronic informed consent is the consent modality used most commonly by PPRNs, with over half reporting that this method is being used currently or planned for the near future. One PPRN stated that an electronic strategy “aligns with the direction of healthcare, particularly electronic health records, and remains familiar to a generation of patients that is, and will continue to be, much more computer- and app-savvy.” The remaining PPRNs are currently still developing their consent procedures, seeking IRB approval for consent protocols and materials, or did not report their consent-related activities in their quarterly progress reports.

Consent development process. To develop consent strategies and language, several PPRNs used committees and meetings, either with internal and external stakeholders or other PPRNs. For example, one PPRN convened a group of stakeholders to research, review, and approve all consent materials before they are submitted to the IRB. PPRNs noted that staffing patients on these work groups was particularly helpful in ensuring comprehension among patients with low literacy and simplifying the consent process overall.

Use of tiered consent. Some PPRNs have incorporated various levels of tiering into their consent processes. In one PPRN, patients who consent to participate in a study are then given the choice to agree or disagree to three additional levels of consent for the study team to receive their medical records, receive their genetic test results, and use their information in future IRB-approved studies. This tiered consent concept was utilized by several other PPRNs, which commonly offered consent options related to data usage and participation in similar and future studies and provided a means for participants to change their consent choices or withdraw consent at any time.

Future goals. Of the PPRNs that have employed electronic consent, many have expressed interest in bolstering these modalities through social media, which aligns with most PPRNs’ focus on online portals and social media as a means to recruit patients into their network and into future research studies. Similarly, a majority of PPRNs with electronic consent protocols expressed plans or future interest to integrate the consent process with EHRs and patient portals. According to one PPRN, the growing familiarity with these tools within their target population makes these linkages particularly desirable and facilitates the potential for improved future processes.

PPRNs are also bridging their consent procedures into potential opportunities to expand data collection and research. One PPRN is exploring leveraging its existing e-consent platform, which is currently used to store patients’ protected health information and identify consented patients for research, to include PRO survey submission capabilities. Expanding PRO integration was a focus for several other PPRNs that opted for electronic or online consent, with one PPRN noting that the electronic platform provided a flexible tool for incorporating additional components.

In summary, CDRNs and PPRNs have taken efforts to improve informed consent processes. Some CDRNs have developed consent templates, while a few have developed innovative tablet-

based apps to facilitate enrollment and informed consent and have devoted particular attention to patient comprehension of informed consent information. PPRNs are making even greater progress developing electronic consent processes, many of which feature tools that allow patients to consent to various forms of data collection and uses of their data. PPRNs are also beginning to leverage social media and patient portals to reach additional patients and to strengthen opportunities for data collection.

While many networks have laid at least some groundwork for making progress during Phase I, differences in approaches between institutions within a CDRN due to custom, concerns about patient vulnerability, or differences in interpretation of federal regulations may have slowed some of this work within networks. Moreover, some of this work may also have been prioritized for Phase II, given the limited number of interventional studies launched during Phase I. At the PCORnet level, participants are eager for additional opportunities to engage around this topic to address current challenges.

Enrolling Patients into Cohorts

Approaches for Identifying, Recruiting, and Retaining Patients

CDRNs and PPRNs systematically identified patients for enrollment into disease cohorts by developing computable phenotypes as part of their Phase I activities.¹⁰¹ At the PCORnet level, the Computable Phenotype work group developed a resource to facilitate CDRN and PPRN development of computable phenotypes. Specifically, the work group canvassed all CDRNs and PPRNs for their current definitions of rare and common disease cohorts and provided technical assistance via discussion-based webinars in June and July 2015 to help CDRNs and PPRNs to provide their definitions in a standardized and comprehensive format. As of the end of Phase I, the work group had begun actively working with the Coordinating Center to develop a library of phenotype and cohort definitions that will be accessible through the PCORnet Central Desktop.

CDRN approaches. CDRNs used one of two different approaches for recruiting patients. Many CDRNs employed a “screen and hold” strategy in which potentially eligible patients were identified through scans of their EHR data and subsequent surveys but were kept in a database with the intention that the CDRN would begin active enrollment once an actual study became available. Other CDRNs directly recruited and enrolled patients into disease cohorts. Some CDRNs in the latter group partnered with clinics or individual clinicians in health systems and used an on-site recruitment process to directly enroll and consent patients into cohorts; others used electronic recruiting approaches through portals or patient networks using software like

¹⁰¹ A computable phenotype is a clinical condition, characteristic, or set of clinical features that can be determined solely from the data in EHRs and ancillary data sources and does not require chart review or interpretation by a clinician (Richesson and Smerek, 2014).

MyChart and REDcap and found them to be particularly effective. CDRNs with multiple distinct partners allowed each of their partnering institutions to use whichever recruitment strategy they felt would optimize recruitment.

Several CDRNs that were actively recruiting and enrolling patients into cohorts indicated that in-person enrollment at clinics and community health centers were effective because of strong, existing relationships between providers and patients. Respondents felt that this strategy was the most effective at enrolling individual patients but was labor intensive. In contrast, “online methods” were described by one CDRN as having a lower success rate per person but produced the largest number of enrolled patients in the aggregate. Most CDRNs reported that they were assessing the performance of their recruitment strategies, but results were often too preliminary to suggest any changes in approach.

PPRN approaches. PPRNs used a greater range of approaches to recruit patients than did CDRNs. PPRNs typically enrolled eligible patients into a registry through multiple outreach channels. Social media (e.g., Facebook, Twitter, etc.) and Internet-based methods (e.g., YouTube, web-based pop-ups, Google ads) were often used to attract potential enrollees, who would then receive follow-up emails encouraging them to enroll in the registry. Email blasts using patient listservs that were maintained by advocacy organizations were also used, but they were reported as having limited impact on recruitment by some respondents. Alternatively, respondents reported higher recruitment success when using a targeted email strategy in which messaging was tailored to specific patient subgroups. PPRNs also widely reported the use of websites to provide patients with information about the PPRN, the disease registry, and other research-related activities.

Many PPRNs formed partnerships with patient advocacy groups, clinics, or hospitals and leveraged these relationships to recruit potentially eligible patients. In addition to asking these organizations to send emails and reminders about PPRN registries or studies, some PPRNs conducted in-person outreach by making presentations at these organizations. PPRNs only occasionally reported using print ads or public service announcements to recruit patients, due to their high cost and the perception that these methods had a more limited reach.

PPRNs described a number of recruitment strategies as being effective. Many respondents reported that social media was their most effective recruitment tool. These PPRNs reported subsequent increases in registry activity after conducting recruitment campaigns on Twitter or Facebook. Another online recruitment activity that was described by some PPRNs as being effective was using advocacy organizations to distribute recruitment notices through email listservs. This strategy was most effective when customized messaging was used to target specific subpopulations.

A number of PPRN respondents noted that there was no “magic bullet” that guaranteed high enrollment rates; rather, they thought it was important to pursue a wide variety of recruitment strategies that would ensure patients’ continued exposure to different types of messaging. As demonstrated by Table 6.2 below, PPRNs employed an increasing number of recruitment

strategies over the course of Phase I to achieve their enrollment goals, with almost all PPRNs using a diverse mix of methods for recruitment by May 2015.

Table 6.2. PPRN Self-Reported Use of Recruitment Strategies (by Reporting Period)

Strategies	June 2014	September 2014	January 2015	May 2015
Email	2	6	16	18
Events	7	6	12	16
Mailed letters	0	1	8	9
Newsletters	3	3	16	16
PPRN website	4	4	14	15
Partner organization recruitment	0	5	11	11
PPRN partner website	2	2	11	11
Phone calls	0	2	8	8
Social media	7	7	16	18

NOTE: The quarterly progress reports used different methods for capturing this information starting in January 2015, so the data may not be completely comparable before and after this date.

Strategies used by CDRNs to retain patients varied by network. CDRNs that were not formally enrolling patients into cohorts had not developed patient retention plans. The remaining CDRNs generally tried to provide patients with additional benefits or demonstrate that enrolling into the cohort provided additional value above and beyond their usual participation in their own medical care. Examples include disseminating research findings in ways that patients can understand easily, asking for patients’ input on CDRN activities (including research topics), and providing a forum in which patients perceive that their voices are heard.

PPRNs reported a broad range of activities aimed at retaining patients who enrolled in their network. One of the most common approaches was to provide online tools through PPRN websites that were designed to keep patients engaged with the PPRN. Examples include frequently updated patient dashboards that allowed for customized data presentations (e.g., comparison of individual patient information to that of other patients with the same condition), social platforms that enabled patients to interact with their peers, and applications that helped patients to self-monitor symptoms (some of which were also available through mobile devices). Additionally, many PPRNs reported trying to keep patients engaged by staying in regular contact with them through email updates, providing opportunities for patients to provide input on PPRN research activities, and conducting periodic brief surveys (which also allowed PPRNs to collect additional data). Surveys were sometimes accompanied by raffles or other incentives.

Implementation Challenges

CDRNs reported two main enrollment challenges. First, some networks reported delays getting IRB approval for their studies due to the complexities of enrolling patients across multiple sites and using novel (electronic) consent techniques. Some also indicated that their electronic consenting and enrollment approaches, particularly CDRNs that were accustomed to

recruiting patients in person, were more difficult to integrate into clinical workflows than originally anticipated. Second, CDRNs reported that variations in their EHR search tools and other capabilities limited their ability to effectively and efficiently identify patients with the conditions of interest.

PPRNs generally described a distinct set of patient recruitment problems. Many PPRNs cited time, staffing, and resource constraints that hindered their ability to meet recruitment goals. Several PPRNs recalled that explaining to patients what PPRNs were, how they operate, and their intended impact on research was a time-consuming process. Identifying and recruiting patients was also reported as more difficult for PPRNs that had to “compete” for patients with other registries, for PPRNs that sought patients in healthier stages or phases (who may not want to participate in patient-related activities), and for rare-disease PPRNs generally. Finally, some PPRNs had difficulty achieving diverse cohorts or even determining whether or not their cohorts were truly representative of the population with the condition, due to limitations of existing epidemiological data on the affected population.

Most CDRNs and PPRNs reported that it was too early to comment about whether or not they would have problems with patient retention. One network that was in existence prior to PCORnet indicated that one of its issues was that patients who were not symptomatic tended to be less responsive to surveys or emails, leading to lower retention of healthier patients.

Progress Enrolling Patients into Cohorts

CDRNs reported their progress recruiting patients into each of three required cohorts in their Phase II funding applications. Overall, CDRNs enrolled nearly 17 million patients into obesity cohorts, 2.7 million patients into common-condition cohorts, and nearly 27,000 patients into rare-disease cohorts (Table 6.3). Comparable totals for PPRNs were not available. Through interviews with PPRN patient engagement leads, the evaluation team learned that about half of PPRNs had not met and were not on track to meet their recruitment goals by the end of September 2015.

As of the 14-month quarterly reporting period, many CDRNs had not surveyed one or more of their cohorts. As a result, it remains too early to tell the extent to which patients enrolled in CDRN cohorts are willing to participate in PCORnet research—one of the major goals of the mandatory patient survey.

Table 6.3. CDRN Patient Enrollment Totals (Self-Report)

	Total Patients Enrolled	Minimum	Maximum
Obesity cohort	16,698,723	215,316	4,900,000
Common condition cohorts	2,700,602	72,000	959,278
Rare condition cohorts	26,299	1,123	5,141

SOURCE: PCORI, undated.

In summary, CDRNs have enrolled or, at a minimum, have identified large numbers of patients with conditions of interest that could be enrolled in future CDRN or PCORnet-wide research studies. Strategies for recruiting patients varied across CDRNs and PPRNs and included both in-person recruitment in clinic settings and electronic recruitment. PPRNs used a broader set of strategies, including social media and Internet-based methods, and engaged both providers and advocacy groups in these efforts. PPRNs, in particular, pursued a variety of ways to keep patients engaged through participation in network activities and novel uses of their patient portals. Recruitment challenges included explaining the purpose of PCORnet, IRB approval, competition for patients without research initiatives, and recruiting diverse patients. It is unclear what proportion of these patients are willing to participate in research, due to slow progress surveying patients as of the writing of this report.

Building Research Infrastructure: Summary of Key Themes

Rapid streamlining of IRB processes. CDRNs rapidly developed or expanded both reliance agreements and central IRBs to streamline IRB review processes during Phase I; two were actively implementing IRBShare. The speed of implementation highlights the intense engagement efforts undertaken by sites locally. However, many stakeholders acknowledged that the real test for multi-site IRB review protocols will come when CDRNs have to review actual study protocols. Differences may then emerge as IRBs grapple with definitions of “minimal risk” or deciding how informed consent should be handled in different types of studies. Ultimately, these disagreements may cause some sites to avoid ceding reviews and may limit the potential efficiency gains from implementing these models. Efforts under way to move to a common multi-site IRB model for PCORnet may enhance the efficiency of IRB review processes in the long run but could be disruptive to individual CDRNs in the short term. Moreover, differences in IRB review models among CDRNs provide opportunities to study the relative effectiveness of different approaches; however, PCORnet will have to weigh the value in pursuing these “learning opportunities” against the potential efficiency gain from using a common approach. Regardless, participants expressed interest in a forum in which to discuss ethics and regulatory issues and to take advantage of the collective wisdom of PCORnet.

Moderate amount of progress improving informed consent. Several CDRNs developed consent templates for use within their CDRN, while a few have developed tablet-based apps in pursuit of a transformative approach for conducting informed consent processes. PPRNs are making substantial progress developing electronic consent applications that allow patients to customize their consent for various types of data collection and their uses. A fair number of CDRNs have prioritized informed consent work for Phase II, given the limited number of interventional studies launched during Phase I. Differences in approaches between institutions within a CDRN due to custom, concerns about patient vulnerability, or differences in

interpretation of federal regulations presented some challenges. Both CDRNs and PPRNs are eager for additional opportunities to engage around these challenges in Phase II.

Some CDRNs may be struggling to implement new recruitment and consent approaches that minimize burden on clinic staff. Addressing this challenge in ways that clinicians find acceptable will be critical for enrolling large numbers of patients into future PCORnet trials. It remains unclear to what extent CDRNs and PPRNs can move away from the “traditional” model of in-person consent administered by physicians to electronic consent approaches offered in clinics or remotely. Evidence from ADAPTABLE and additional PCORnet demonstration projects will help to determine whether more efficient recruitment approaches can be acceptable to clinicians, patients, and IRBs.

Enrolling patients into condition cohorts. Most CDRNs appear to have developed the important foundational capability to screen patients for clinical trial eligibility using standardized data contained in their DataMarts. However, many CDRNs that used a “screen and hold” strategy may not have actually enrolled large numbers of patients into disease cohorts. For their part, PPRNs have developed, tested, and deployed a wide range of outreach methods to identify and recruit members of their target populations into their networks. While many PPRNs had previous experience conducting outreach using multiple modalities, PPRNs are using PCORnet funding to develop or expand their patient portals to enhance their engagement and data collection activities. Despite these efforts, half of PPRNs reported difficulty meeting their enrollment goals.

It remains unclear how effective CDRNs’ and PPRNs’ recruitment strategies are until they are tested in an actual study. Also unclear is the degree to which CDRNs and PPRNs will be able to recruit sufficient volumes of patients to support PCORnet research, since data from CDRN cohort surveys indicating patients’ willingness to participate in PCORnet research were not available as of the writing of this report.

7. Developing a Culture of Collaboration

One of the key unanswered questions at the start of Phase I was whether or not a network of 29 independent entities—most of whom had little history of collaborating with one another—would successfully come together in pursuit of PCORnet-level goals. The ability of PCORnet to conduct research on the scale envisioned by PCORI requires CDRNs and PPRNs to engage in cross-network research that goes beyond their locally defined research priorities. Moreover, to quickly develop research capacity, CDRNs and PPRNs would need to collaborate closely to overcome the governance, operational, and technical challenges that would arise in the course of implementing PCORnet’s infrastructure. To assess progress in developing a culture of collaboration, we examined

- the scope and nature of collaborations that emerged during the course of Phase I
- barriers and facilitators to collaboration
- participants’ overall experience with these collaborations.

Approach to Fostering Collaborations

The Coordinating Center was the main entity within PCORnet that took steps to foster collaborations between networks. Its initial strategy centered around the task forces and, later, the PCORnet demonstration projects. By participating on the task forces, which were designed “to do much of the work of the network” as described by PCORnet leadership, it was anticipated that CDRNs and PPRNs would learn firsthand the unique areas of expertise of each network and that these informal interactions might then form the basis of future collaborations. As the demonstration projects took on a larger share of CDRNs’ and PPRNs’ cross-network activities, these projects provided another opportunity for CDRNs and PPRNs to come together for a common purpose.

The Coordinating Center also used regularly scheduled meetings and other tools to promote collaboration. Steering Committee meetings provided CDRNs and PPRNs opportunities to present their work to one another, and thematically organized breakout sessions allowed CDRNs and PPRNs to discuss specific implementation challenges or share best practices in small-group settings. In addition, during one Steering Committee meeting, the Coordinating Center used a “speed dating” approach in which CDRNs and PPRNs met individually with one another on a rotating basis to give networks an opportunity to identify areas for potential collaborations. The Coordinating Center also used a variety of standing conference calls with CDRNs PIs, PPRN PIs, and project managers to provide another opportunity for engagement. Finally, the Coordinating Center’s PMO created best practice sharing sessions that were held biweekly to provide a forum in which networks could discuss innovative strategies for specific activity areas

and to encourage dissemination of best practices throughout the network. Topics included clinician and patient engagement strategies, frameworks for data access use and protections in clinical research, streamlining IRB review for multi-site studies, and other issues relevant to network-based research. To strengthen these efforts, the PMO also established a space on PCORnet’s collaboration website (known as Central Desktop) so that CDRNs and PPRNs had a platform dedicated solely to the purpose of facilitating collaborations.

Implementation Progress

Level and Nature of Collaborations

Most CDRNs and PPRNs actively sought out and developed collaborative relationships with other networks within PCORnet. Figure 7.1 and Figure 7.2 display the number of networks with which CDRNs and PPRNs collaborated, respectively, as of the May 2015 progress reports (month 15 of Phase I). CDRNs reported collaborating with between 5 and 16 other networks (an average of 11 networks per CDRN), whereas PPRNs reported collaborating with between 3 and 12 other networks (an average of 7 networks per PPRN). Both CDRNs and PPRNs tended to report more collaborative relationships with CDRNs than with PPRNs, most likely because CDRNs would be more likely than PPRNs to provide potential partners with access to large populations of patients within diverse clinical conditions and, thus, many more potential research opportunities.

Figure 7.1. Number of CDRN/PPRN Collaborations Self-Reported by CDRNs

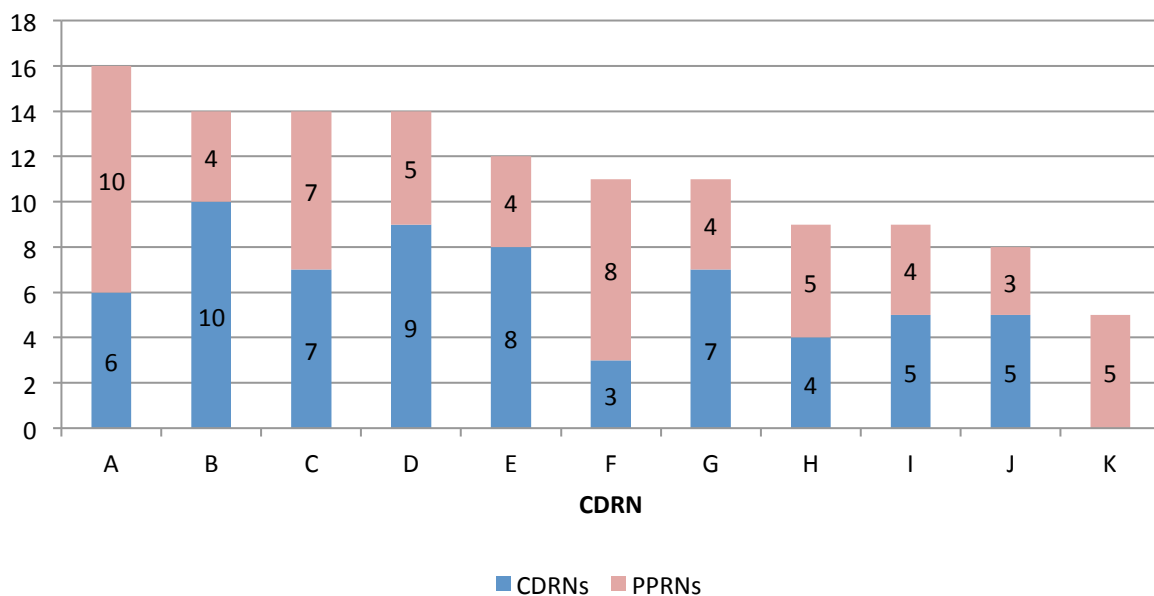
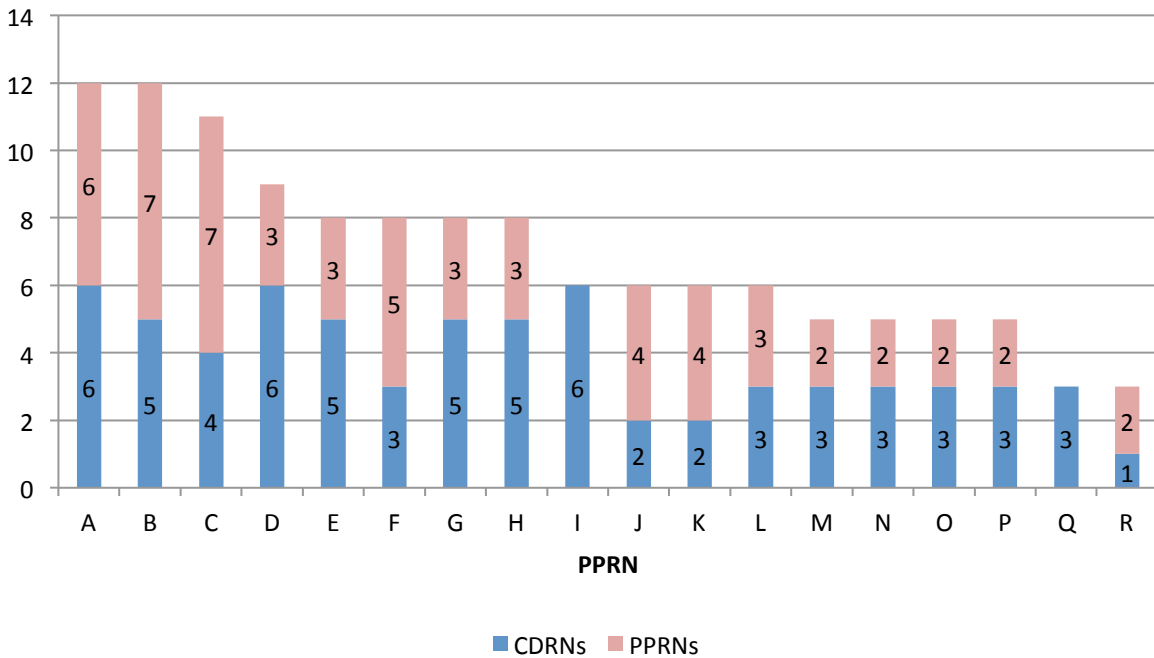


Figure 7.2. Number of CDRN/PPRN Collaborations Self-Reported by PPRNs



The nature of these collaborations included a wide variety of activities that supported the full scope of organizational, research-oriented, and stakeholder engagement activities employed by the networks. Table 7.1 below summarizes the types of collaboration activities reported by CDRNs and PPRNs and the number of networks that reported each activity.

Table 7.1. Collaboration Activities Reported by CDRNs and PPRNs

Collaboration Activity	Number of CDRNs	Number of PPRNs	Total Number of Networks
Data linking and data sharing	10	8	18
Co-referral or patient recruitment	5	12	17
Development or validation of computable phenotype	5	12	17
Developing or co-participating in studies, clinical trials, abstracts	9	6	15
Co-enrollment	3	10	13
Data standardization	6	6	12
Assistance with IRB issues (application, consents, protocols)	3	8	11
Developing engagement strategy or sharing best practices on engagement	4	6	10
Co-development of or assistance developing patient tools or surveys	3	6	9
Assistance with policy or governance development	1	4	5
Co-presenting at conferences, webinars, etc.	1	1	2
Clinician engagement	1	0	1

As illustrated in Table 7.1, the types of collaborations pursued by CDRNs and PPRNs differed in a few key ways. Whereas almost all CDRNs reported that data linking and data

sharing comprised at least one of their collaboration activities, both co-referral/patient recruitment and computable phenotype development/validation¹⁰² were reported as the most common collaboration activities among PPRNs. In addition, CDRNs appear to be more likely to seek and support collaborative relationships that involve data sharing and preparations for clinical trials while reporting fewer relationships with networks that support recruitment and other research preparation activities. Conversely, PPRNs are very highly engaged in partnerships involving recruitment and enrollment activities and reported slightly lower rates of collaboration for data preparation activities, including data standardization. Neither CDRNs nor PPRNs reported high rates of collaboration for research dissemination activities (e.g., webinars and conferences) or clinician engagement.

Additional details about the nature of collaborations between networks employed by CDRNs and PPRNs are described in the sections below.

Collaborations Around Patient Identification, Referral, and Recruitment

Some PPRNs collaborated with CDRNs to identify patients in the CDRNs' health systems using a computable phenotype. In some cases, CDRNs were able to go beyond identifying the patient population of interest and actually contacted patients to ask if they would like to be connected to relevant patient groups or research opportunities.

A few respondents discussed plans for co-enrolling patients jointly in CDRN and PPRN networks. In these arrangements, CDRNs typically were responsible for extracting EHR data, while PPRNs would lead the collection of PROs.¹⁰³ Some networks viewed this type of co-enrollment model, in which researchers would have access to both EHR and PROs, as critical to enhancing the value of CER. As stated by one respondent:

All those data that you can get from cross-referencing ICD-9 codes miss the data about patient symptoms and patient well-being and quality of life. . . . [A]ny study that can be done within a CDRN would be so much more enhanced by being able to include data reported directly from patients and the easy conduit of getting that patient-reported data is through the PPRNs. . . . And so when you talk about building a comparative effectiveness research infrastructure nationally, what you need is patients that are in both PPRNs and CDRNs so that those two sets of complementary data can be pulled together for research purposes. . . . the way to [build a CER infrastructure] isn't to have CDRNs and PPRNs working in parallel, but it's to have them working in a way that's completely enmeshed.

¹⁰² A computable phenotype is a clinical condition, characteristic, or set of clinical features that can be determined solely from the data in EHRs and ancillary data sources and does not require chart review or interpretation by a clinician (Richesson and Smerek, 2014).

¹⁰³ PROs are defined by the FDA as a report of the status of a patient's health condition by the patient or his or her proxy without interpretation by a clinician or anyone else.

Other PPRN-CDRN collaborations included cross-promotion (e.g., including information about PPRNs to CDRN patient-facing websites), and, in at least one case, a CDRN used a PPRN’s patient registry to screen and recruit patients for a research study.

As indicated in Table 7.1, these collaborations were concentrated within a relatively small number of CDRNs that worked with multiple PPRNs. Ease of use of CDRN resources was noted as helpful by PPRNs. For example, several PPRNs appreciated one CDRN’s use of a template for data requests to identify potential PPRN enrollees within the CDRN’s network.

Collaborations Around Data Transformation and Data Portals

Networks sometimes collaborated on the data transformation tasks that were required for the common data model. Several cited preexisting data partnerships, such as already sharing a platform or previous work together implementing data standards, such as i2b2.¹⁰⁴ One CDRN, in particular, collaborated with several PPRNs, resulting in a small learning community around data transformation issues. Respondents also described sharing programming code and discussing challenges with other networks that had used an alternative common data model prior to Phase I and, therefore, that had faced similar challenges implementing the PCORnet common data model.

A few rare-disease PPRNs collaborated with one another to share their experience around building patient registries and web portals, including their experience with specific vendors. This cross-network activity was described as leveraging their “common perspectives” as patient advocates working with rare-disease communities.

Natural collaborations also formed among networks that were using data from the same health system (e.g., users of the University of California Research eXchange [UC ReX]), who described coordinating efforts around patient identification and recruitment. For PPRNs, one important benefit of working with health system data was having access to large and more diverse groups of patients—including, but not limited to, racial and ethnic diversity—than would otherwise be possible through their current recruitment methods. Coordination between PPRNs and CDRNs around the use of health systems’ data focused on patient identification and recruitment in the first year of PCORnet, but one respondent described hopes for integration of PRO data into health systems’ data as the longer-term goal.

¹⁰⁴ I2b2 is an NIH-funded National Center for Biomedical Computing based at Partners HealthCare System. The Center has developed a software program that organizes and transforms clinical data into tables. The central table contains many different “facts” or observations on a patient, such as patient and provider numbers, the concept that was observed (e.g., diagnosis, medication, procedure, or laboratory test), the value of the concept (e.g., diagnosis code, procedure code, laboratory test result), start and end dates, and other elements.

Collaborations Around Data Linking and De-Duplicating

Many networks collaborated on strategies to uniquely identify patients within and across multiple data sources in a way that avoided the transfer of protected health information. Collaborations around linking and de-duplicating data were prioritized for several reasons. For CDRNs with multiple local health systems, there was a need to accurately identify patients as they moved between health systems to eliminate duplicate records. De-duplication of CDRN and PPRN datasets that may contain the same individuals was also a concern.

Many networks discussed collaborating to identify a viable solution to this problem. One effort used a hashing approach that led to a collaboration with multiple CDRNs and PPRNs, as described previously in Chapter 5, “Building PCORnet’s Data Infrastructure.” Other networks discussed a “global ID” approach to linking data across networks.

Collaborations Around Research Studies

Although the goals of Phase I did not include initiating research studies beyond the PCORnet demonstration projects, nearly half of all networks (nine CDRNs and six PPRNs) were engaged in research studies that were either planned, in progress, or completed that were the results of collaborations between networks.

One completed study was a collaboration between a PPRN and a CDRN that used CDRN data to understand the types of specialists that patients with a certain condition were seeing, which helped the PPRN strengthen its clinician-focused educational efforts.

Most other research was in formative or early stages. Two CDRNs are collaborating on a clinical trial involving heart failure patients that will leverage Medicare claims data to ensure complete follow up of patient outcomes. PPRN respondents discussed ideas and plans for using CDRN data, such as understanding whether or not practice guidelines are followed based on analysis of EHR data. Several CDRNs that selected diabetes as their common-condition cohort (one of the three patient cohorts that all CDRNs were required to establish during Phase I) are in the early stages of collaborating on diabetes-related projects. A group of five CDRNs are also laying the groundwork for a study on sickle cell disease.

Another early phase of research collaboration occurring between PPRNs and CDRNs was proposal development. One collaboration between two CDRNs and a PPRN resulted in a proposal to study the safety of joint implant devices. Collaborations between pairs of CDRNs have resulted in three other proposals that were submitted to PCORI by the end of Phase I. Although it was not clear from respondents’ descriptions whether or not this work was making use of PCORnet infrastructure, it seems that newly formed relationships and recognition of the potential value in collaborating were spurring ideas for ongoing research partnerships.

Broad-Based Collaborations

Other collaborations between networks emerged that were less task- or topic-focused but, rather, were based on broad commonalities or shared challenges. These types of collaborations allowed networks to learn from peers and, through discussion of common goals, work through ideas about goals, vision, and road maps for their individual networks and for PCORnet as a whole.

Many networks came together around an interest in pediatric populations. Some of the networks were already affiliated with one CDRN that focused on pediatric populations, which was a valuable partner because many children with rare conditions sought care at the CDRN's hospitals, providing a limited set of health systems that would contain a large majority of pediatric rare diseases. The CDRN's researchers were described as very interested in collaborating and issued broad invitations to other networks to meet with them. While cross-network collaborations around pediatric populations were mostly within the CDRN, there was at least one example of a collaboration outside of the CDRN in which non-pediatric-focused PPRNs collaborated with pediatric-focused networks based on an interest in younger patients with their conditions of interest and to gain from those networks' experience with the research challenges of studying children.

Rare-disease PPRNs worked together as well to answer questions and address shared PCORnet issues, but they also engaged on topics broader than PCORnet. For example, PPRNs that were part of one broad-based collaboration discussed strategies for dealing with industry requests and the FDA.

Networks reported that they found themselves collaborating with others in PCORnet around general project management issues, such as managing project milestones and responding to requests from PCORnet leadership. These networks also found it helpful to have project managers and non-PI project staff meet to work through details of the Phase II application process. One group of PPRNs described similar informal collaboration of project staff through monthly conference calls in which they would cover a wide range of issues. This group seemed to use these less formal, supplemental interactions—"off-line dialogue," according to one respondent—as opportunities to work through what was happening with PCORnet, such as discussing reactions to policies before returning comments. One CDRN described attending several PPRNs' management meetings in order to discuss the capabilities of the CDRN and make connections about how their resources could help accomplish the PPRN's goals. Finally, some CDRNs reported attending each other's management calls to share best practices and experiences around challenges that both CDRNs had encountered.

Barriers to Collaboration

Despite the high level of collaboration activity, respondents identified numerous barriers to collaboration during Phase I, described in detail below. In general, most respondents endorsed

the importance of collaboration but lacked guidance and resources necessary to build functional collaborations. Respondents discussed confusion about models of collaboration and the value proposition for collaboration from various perspectives. In the face of many other responsibilities for building infrastructure and meeting milestones, collaborating with other networks sometimes received lower priority.

Lack of vision or examples to guide collaboration. Some networks struggled with understanding how and why collaboration could contribute to their organizational goals and to PCORnet as a whole. Respondents discussed how it was unclear at the outset how PPRNs and CDRNs would make use of their data to create synergies. One respondent felt that the lack of articulation of a clear vision and potential for collaboration within PCORnet stymied partnerships and other efforts to develop the broader collaborative network.

Participants also had several specific questions about how PPRNs would participate as partners. For example, how would PPRNs be involved in clinical trials? The case of rare-disease PPRNs created additional collaboration challenges because their patient populations were so small (relatively speaking), and often scattered geographically, that networks struggled with imagining how they would fit into eventual clinical trials.

Respondents said that sharing of illustrative use cases was helpful to networks' understanding of the potential and models for collaboration, but the lack of a "clear pathway or a clear model" for CDRN-PPRN collaboration remained a barrier to collaboration. In the absence of PCORnet-wide models, some sites described instituting local policies to guide collaboration.

Short time frames. Most respondents reported that limited time was a major barrier to collaboration. Within the finite time and resources dedicated to Phase I, many networks described their primary focus as building infrastructure. This was especially important to new networks that were working through internal governance and operational issues. A few networks described how they justified prioritizing building infrastructure over collaboration by emphasizing the dependencies between different components of PCORnet. One PPRN respondent's comment was representative. After outlining the three central tasks that the respondent conceptualized as critical to infrastructure-building (creating a portal, recruiting patients, and obtaining sustainability funding), the respondent commented, "So collaboration sounds great, except it's a distant fourth [task]. Because if you don't have the first three [components], you don't have anything and you can't collaborate if you don't have those three."

Beyond the focus on other tasks that were perceived as higher priority or more necessary than collaboration in the early stage, networks also reported that the required deliverables for Phase I, overall participation in PCORnet, and network administration were all very time consuming. Networks struggled to find the right balance between the needs of their local network and the expectations of PCORnet. One respondent referenced the need to fulfill over 100 deliverables pertaining to each CDRN and PPRN's own local activities and, thus, a limited incentive to participate in network-wide activities. Another respondent suggested that CDRNs

struggled significantly with the allocation of resources for the things they had promised locally versus what the national network needed.

The tight timelines interacted with the shifting scope of work throughout Phase I to create additional barriers. For example, one network described how each time it needed to fulfill queries as part of planning for PCORnet demonstration projects (which often required additional data mapping because the common data model did not contain all necessary data elements), it pushed the network's timeline on other deliverables. Toward the end of Phase I, networks felt even more pressed for time, with the competing responsibilities of completing Phase II applications and demonstrating productivity during Phase I. Beyond the time required for Phase II applications, one respondent noted that the potential for networks to lose funding at the end of Phase I created a mood of competition among the networks, making resources feel all the more scarce.

A final way in which lack of time was a barrier to collaboration related to how networks conceptualized meaningful collaboration. Some networks commented on the time required to create a good partnership and complained that neither CDRN nor PPRN members had time for the type of exploratory conversations that would have been the necessary foundation for a partnership.

Inadequate funding to build infrastructure and engage in robust collaboration. Many respondents described ways that the level of funding available to networks in Phase I was a barrier to collaboration. In general, respondents felt that the level of funding might be adequate to support building the network but was not sufficient to also enable robust collaborations outside of infrastructure-building. For example, when discussing the adequacy of the budget, one PPRN described many activities not even being considered because they required much more than current funding levels could support. In this way, funding levels stifled or reduced the scope of collaborative work between networks. One network that seemed to struggle to stay within its budget described a situation in which limited resources were spread too thin across its many partners. When collaborations involved “networks of networks” that bring many people to the table, this network struggled with how to meaningfully engage partners without always involving—and needing to compensate—everyone.

Other CDRNs and PPRNs recognized that the Phase I funding was part of the life cycle of PCORnet and described how the next necessary step is getting additional funding support in order to conduct studies. A few CDRNs took an alternate and more immediate approach to raising funds to cover the additional costs of collaborating by requesting payment from PPRNs for data requests. One particular CDRN that adopted this model felt that it had already given in-kind support to PPRNs that went beyond the PCORnet funding. According to one CDRN respondent, payments that they received to identify patients were relatively small—between \$10,000 and \$20,000—but were enough to cover the costs of doing the work. (When PPRNs discussed these requests for payment, they cited the higher figures of \$50,000 and \$60,000,

although it was unclear whether they paid these fees and how many CDRNs requested payment for their assistance.)

Lack of a value proposition for CDRNs to work with PPRNs. A few respondents cited the lack of a value proposition for CDRNs to work with PPRNs. Given the resources involved in collaborating, it was not clear to all networks how collaborations would be mutually beneficial and not simply “one-way collaborations” in which CDRNs provide data and analysis for PPRNs and get nothing in return.

Respondents made a connection between a lack of understanding of the value of PPRNs and two features of the common data model. First, one respondent suggested that not including a place for PPRN data in the first version of the common data model propagated or, at least, did nothing to refute the idea that PPRNs had little to contribute to collaborations. Second, another respondent discussed how the limitations of the first version of the common data model were an obstacle to collaboration because the common data model was not always complete enough to apply the computable phenotypes that would identify the patients PPRNs were interested in recruiting. The value proposition for engaging with rare-disease PPRNs appeared to be least clear for CDRNs; patients with certain rare conditions may lack a reliable computable phenotype and require text searching in the EHR to identify them.

The lack of a clear value proposition for CDRNs to collaborate was keenly felt by PPRNs because there was no mechanism or clear expectation that CDRNs would partner. Respondents noted that the requirement to collaborate was asymmetrical: PPRNs were expected to partner with CDRNs for EHR data linkage, but no parallel requirement was present for CDRNs. Furthermore, CDRNs came into the project with disease cohorts already defined. Therefore, asking networks to collaborate to help develop PPRN disease cohorts may have been perceived as extra work. One PPRN respondent also discussed how difficult it was to find clinician champions to facilitate patient outreach and shared that a requirement for CDRNs to communicate with their clinicians about PPRNs would have been helpful.

Looking forward to Phase II, respondents felt that there was a clearer understanding of and communication around the value proposition for collaborating with PPRNs. Several PPRNs praised Phase II’s explicit minimum requirements for CDRNs to collaborate with PPRNs and the fact that CDRNs needed to show in their budgets that appropriate resources were allocated to fund the work. This change was seen as a positive course correction that networks wished had been in place in Phase I.

Lack of infrastructure and poor communication. Some respondents viewed PCORnet’s approach to fostering collaboration as too passive and indicated that only limited amounts of information were available to networks to support collaborations taking root. One respondent reported widely felt skepticism about the approach of “providing space” for collaborations that were then expected to develop naturally. A specific example of the lack of infrastructure: There was no centralized list of networks that described the key implementation strategies they were using or their areas of expertise. One respondent mentioned the lack of communication tools to

connect networks, especially in the early stages, as an obstacle to early peer learning and informal collaboration regarding best practices for setting up networks. Some participants viewed this as a missed opportunity for leveraging the enthusiasm of and willingness of participants to engage with peers.

In addition, although task forces were a structural feature of PCORnet that some respondents thought could have helped to foster greater collaboration, CDRNs and PPRNs noted variation in the level of collaborative culture across task forces. For example, one CDRN noted that the Ethics and Regulatory Task Force did not feel that it had much problem-solving work to do in the early stages of Phase I, so participants did not view the task force as contributing effectively to collaborations around human subjects issues.

CDRNs and PPRNs held mixed views on the Coordinating Center's attempts to connect networks through the "speed dating" activity. For example, one respondent described how, if after five minutes of introductory conversation, no obvious areas of overlap appeared, there was not a useful next step; the chance for collaboration seemed to just end there. This respondent had the sense that most collaborations occurred outside of the Coordinating Center's oversight. Others viewed the "speed dating" event as a useful opportunity to make an introduction, even if it did not lead to a collaboration.

Several CDRNs and PPRNs identified the potential value of condition or topic affinity groups to help foster collaboration. Although at least one participant perceived resistance to creating factions or blocks of networks within the larger network, others thought that creating groups based on common interests would foster synergies, including the possibility of forging a larger research agenda around a topic area rather than merely conducting individual unrelated projects. Examples of interest areas cited by respondents that would be candidates for forming groups included safety net providers/populations, pediatric neurological diseases, and specific treatments.

Varying degrees of readiness of CDRNs and PPRNs to collaborate. Respondents described variations in the capabilities and experience of networks at baseline, especially among PPRNs, putting some in a better position to collaborate than others. One respondent spoke generally about this issue by citing differences in the maturity of each network, which may impact a network's willingness to engage in collaboration. This respondent noted that the CDRNs are in different places in their maturity and may not see value in collaborating with less-mature CDRNs. Similarly, some participants believed that new or less experienced PPRNs were so focused on getting their networks up and running that they lacked time or capacity to collaborate with other networks. One respondent had the impression that the PPRNs that did engage in collaboration were more established, and some had preexisting partnerships with CDRNs.

Respondents were more specific about how PPRNs differed in their capacity and readiness to collaborate with CDRNs on research. Data capabilities were one area of variation. For example, some networks were just starting registries, while others had robust and long-standing registries.

Most PPRNs also had limited clinician involvement and data informatics infrastructure, which caused some to struggle with data issues more than others.

A few respondents noted the challenges of collaborating with relatively underdeveloped PPRNs. One respondent noted greater difficulties collaborating with new networks that are “building from scratch” than with more established PPRNs. Another respondent faulted the approach of some PPRNs that were trying to collaborate with CDRNs. Rather than appeal to CDRNs with a blanket request for help, this respondent thought that PPRNs would do better to approach CDRNs with actual research questions that were more likely to appeal to the strengths of the prospective partners.

Another dimension in which PPRNs varied was their orientation around research. One PPRN participant noted that his group was “obsessed” with improving recruitment to its portal and found it difficult to step away from that paradigm to think in terms of research. In general, PPRNs without research experience reported fewer collaborations around research.

Facilitators of Collaboration

Several factors facilitated collaboration between networks during Phase I. The most important facilitators, by far, were preexisting collaborations or working relationships and institutional relationships. One pair of collaborators already shared a data platform that was based on prior work. These networks applied to PCORnet together and described their collaboration as being “written in” as part of the contract. Another pair of collaborators had previously worked together to implement the i2b2 data model. Yet another pair of collaborators were part of the same institution and discussed how this relationship and the ability to leverage institutional resources (e.g., using attorneys to craft data use agreements) facilitated their working together. In cases where prior working relationships did not exist, overlap of clinical conditions between networks helped to promote collaborations. For example, PPRNs involved with epilepsy and mental health both described the advantage of having conditions in common with other networks in their efforts to collaborate.

The PCORnet demonstration projects and the need to apply for funding for future work were two examples of how doing the work of PCORnet created opportunities for collaborations. One PPRN credited the ADAPTABLE trial as facilitating fruitful collaboration around concept development and study design. Other respondents felt that the need for sustainable funding facilitated collaborations by bringing PPRNs and CDRNs together around proposal development.

The application process for Phase II also stimulated collaboration activity. Networks reported working together to develop Phase II proposals. One respondent noted that networks shared drafts, something that the respondent found remarkable, given that Phase II was a competitive process. The same respondent described how planning for the next phase generated many concrete ideas for how CDRNs and PPRNs could collaborate in practical ways in Phase II, such as sharing statisticians or literacy experts.

Participants' Experience with Collaborations

Perspectives on the success of Phase I collaborations varied across stakeholders and ranged from very negative to very positive. In general, participants had different perspectives on CDRN collaborations and PPRN collaborations.

Perspectives on CDRN collaborations. CDRNs reported valuable sharing of knowledge and generally positive experiences regarding their collaborations with other CDRNs. According to CDRNs, these collaborations were useful because some topics that were germane to CDRN challenges were not being discussed within the wider PCORnet group meetings.

While many CDRNs reported that collaborations with PPRNs often involve large commitments of time, one CDRN participant felt that the difficulties identifying and recruiting patients were challenging and that it was “important to start early thinking about these [issues] . . . and if we had the capacity, as we did in Phase I, to do this, we thought it was a good use of our resources.” Another CDRN participant described how the value of PPRN collaboration only became apparent over time. This participant shared how the persistence of a PPRN partner forced them to deal with key recruitment and data-sharing policies at the level of the CDRN institution’s leadership and legal counsel, suggesting that PPRN involvement may have accelerated some processes.

While most CDRNs viewed network collaboration as an area of success during Phase I, one CDRN PI took a different view and suggested that only about half of CDRN PIs were committed to collaboration at the PCORnet level. Some members of PCORnet leadership echoed this view, while others interpreted CDRN attitudes toward engagement as evolving. One member of PCORnet’s leadership believed that the majority of CDRNs had “bought in” to the idea of PCORnet but that many struggled with comprehending the scope of the project and the road map for a national research network. Taking the perspective of a CDRN, another respondent admitted that PCORnet was an untested idea and that at this early stage it was hard to imagine the full potential and identify what trade-offs would be necessary, to which the respondent credited the continued ambivalence of networks.

Some members of PCORnet leadership felt that it was only a small number of CDRNs that were either far more committed to their own local priorities or that had a different strategic vision for the network. From their perspective, it was large, research-focused CDRNs that were the least likely to work toward the vision of PCORnet as a national resource and that tended to view PCORnet as an opportunity to improve their own research program. According to the respondent, the idea of a national resource did not fit within their paradigm for how research was conducted.

These negative views may reflect early perspectives on collaboration that later evolved. PCORnet leadership described collaboration among CDRNs as getting off to a slow start, but expanding over time. One respondent described how the sharing of documents—such as outreach materials, documents about network formation, compensation, data use agreements, and

consent forms—among CDRNs has steadily evolved. First, there was a hesitancy to share drafts or non-final versions of documents or tools. The respondent believed this hesitancy to be less about intellectual property and more about uncertainty or not wanting to risk circulating tools that turned out to have major shortcomings. The respondent also discussed how these attitudes were improving as relationships and rapport grow between networks.

Another member of the PCORnet leadership described how CDRNs, more than PPRNs, have become quite collaborative, writing proposals together and coming together informally to share strategies on how to address Phase I milestones. This finding of “common ground” around challenges related to project deliverables fostered the development of relationships and rapport, which, in turn, resulted in collaborations. This respondent described the current situation by saying, “[The CDRNs] are all friends now. When they have a research project, they call each other. They don’t [only] go to the people that are right next door in their research department.”

Overall, the level of buy-in and engagement among CDRNs was highly variable. Some networks appear naturally inclined to collaboration, while others remained unclear or uncertain about the value proposition for PCORnet to their CDRN. But there was also a sense that attitudes are still evolving and that future opportunities to engage all networks in the vision of PCORnet may be successful.

Perspectives on PPRN collaborations. PPRNs were generally disappointed with CDRNs’ limited willingness to collaborate and support their organizational priorities. One PPRN acknowledged the complexity of CDRNs’ infrastructure-building activities but also expected that they would have been “more willing to meet us halfway.” Another respondent echoed this sentiment by noting that, with one exception, he or she had not witnessed CDRNs “reaching out” to engage PPRNs in partnerships. Another PPRN respondent complained about CDRNs’ inflexibility and wanted to see more opportunities for collaboration with CDRNs beyond co-enrollment.

PPRNs were confused and unhappy when CDRNs asked for payment to run queries to identify patients. PPRNs acknowledged the competing priorities and pressures that CDRNs face and realized that meeting the needs of PPRNs was often lower on the list of CDRN priorities. However, when CDRNs said they were too busy to complete requests without payment, PPRNs questioned the significance of co-existing in PCORnet with the CDRNs.

Despite these challenges, PPRNs reported that rudimentary collaborations with CDRNs were emerging; because partnerships take time to develop, a respondent described collaborative efforts as just getting started. Similar to how practical work products were a general facilitator of collaborations, one PPRN respondent described intensively working with a CDRN to get a project off the ground as a positive collaborative experience.

While PPRNs cited difficulties collaborating with CDRNs, several mentioned collaboration within the PPRN community as an unanticipated and positive result of being involved in PCORnet. PPRNs described sharing best practices around recruitment and retention even though not all PPRNs described themselves as natural partners.

Unlike CDRNs, however, several respondents reported that PPRNs were less able to do the type of cross-network problem-solving and peer sharing that built relationships among the CDRNs. One respondent attributed this failure to find common ground to there being so much diversity within PPRNs, and also to some “strong personalities” that may have gotten in the way of a more cohesive group dynamic. Similar to CDRNs, one respondent noted that some PPRNs are less inclined to participate in PCORnet-wide activities; for those networks, the focus appears to be on building up their own agenda, rather than on contributing to the vision of a national network. According to one member of PCORnet leadership, through most of Phase I, “there were still 18 separate communities of research advocacy groups, rather than . . . a united sense of these 18 organizations making the case for patient-centered research.”

The inability of PPRNs to find common ground and the need to strengthen the role of PPRNs in PCORnet overall were two of the main reasons that prompted the appointment of a third PI to the Coordinating Center to coordinate PPRN activities. The external evaluation team’s interviews were completed shortly after this appointment, and, thus, we were unable to gain the perspectives of participants on the potential impact of this change. Nevertheless, most PPRNs enthusiastically supported this change and are optimistic that PPRNs will play a larger role in all aspects of PCORnet during Phase II.

Collaboration: Summary of Key Themes

CDRNs and PPRNs faced numerous challenges engaging in infrastructure- or research-related collaborations during Phase I. CDRNs and PPRNs noted that the vision surrounding collaborations and the articulation of specific roles CDRNs and PPRNs could play in collaborations was missing for most of Phase I and could have helped to guide collaborations early on. Time pressure to complete each network’s Phase I milestones, tight Phase I budgets, and the lack of a clear value proposition for CDRNs to work with PPRNs so that both parties would find collaborations to be “mutually beneficial” may have limited the ability of some CDRNs and PPRNs to engage in collaborations. Other CDRNs and PPRNs believed that critical infrastructure needed to promote collaborations was lacking and that PCORnet leadership’s primarily passive approach to collaborations, the lack of basic information about networks’ implementation strategies and expertise, and the lack of affinity groups may have undermined collaborative efforts. Differences in networks’ readiness to engage in collaborations and IRB issues may also have created barriers.

Despite these challenges, CDRNs reported collaborating with an average of 11 networks, and PPRNs reported collaborating with an average of seven networks. Collaborations spanned patient identification, referral, and recruitment activities; data standardization and linkage; research studies; and broad-based collaborations around multiple topics. Facilitators of collaborations included prior relationships, institutional relationships, participation in PCORnet demonstration projects, and Phase II requirements involving CDRN and PPRN partnerships. The evaluation

team did not have access to data that could help to assess the value of collaborations, such as new research proposals, funding, or new abstracts or manuscripts. However, since Phase I focused on infrastructure-building, these assessments will be more useful during Phase II, during which networks are anticipated to be conducting numerous research projects.

Some concerns remain among CDRNs, PPRNs, and PCORnet leadership that the level of collaboration activity across the network may be suboptimal. Some CDRNs and PPRNs may not have embraced the vision held by many that PCORnet should function as a national resource. These networks may continue to prioritize local research goals and local collaborations. The number of these networks, by most accounts, is quite small, and attitudes of CDRNs and PPRNs toward PCORnet-level collaboration may be evolving concomitantly with PCORnet's governance model and as the many PCORnet-level work groups accelerate their activities as Phase I comes to a close. Moreover, some participants believe that embracing cross-network collaboration is a gradual process that simply has required most of Phase I to generate visible progress.

As the network heads into Phase II, prospects for greater collaboration appear high. The appointment of a third PI to the Coordinating Center to spearhead PPRN activities may strengthen the role of PPRNs in the network as a whole and help to communicate their value to CDRNs. The requirement for CDRNs to collaborate with PPRNs during Phase II was seen as a positive development by many participants that may have provided the necessary motivation for networks to specify concrete plans for collaboration. Insofar as research projects may further strengthen linkages between CDRNs and PPRNs and draw on each type of network's unique strengths (a view held by both types of participants), the continued progression of PCORnet's data infrastructure may be the most important facilitator of greater collaboration between CDRNs and PPRNs in Phase II.

8. Engaging Stakeholders in PCORnet

The long-term sustainability of a research network like PCORnet requires strategic guidance, active participation, and financial support from a broad set of health system stakeholders. Since the network's inception, PCORI, the Coordinating Center, and individual CDRNs and PPRNs have recognized the need to engage diverse stakeholders in the governance and operations of PCORnet. Five groups whose engagement was considered high priority for Phase I are patients; clinicians; health system leaders; federal partners and funders; and pharmaceutical, medical device, and health insurance industry partners and funders.

Each group played a unique role helping to establish PCORnet during Phase I. Activities included supporting PCORnet and local network governance, developing research topics, participating as research subjects, providing data to support research, and contributing time and resources to develop research capacity. The strategies used by PCORnet to engage stakeholders and the role that these stakeholders played in PCORnet's development are described in further detail in the following sections.

Engaging Patients in PCORnet Governance

CDRNs and PPRNs identified patients to serve on PCORnet-level governance bodies and local (CDRN/PPRN-level) governance bodies. We discuss each of these activities in turn, beginning with PCORnet-level governance.

Approach for Engaging Patients to Serve on PCORnet Governing Bodies

Most PPRNs and CDRNs identified and recruited patients to serve on one or more PCORnet governing bodies, including the Executive Committee, the Steering Committee, the Patient Council, and task forces.¹⁰⁵ Some networks kept abreast of patient participation on PCORnet governing bodies through routine debriefing, but other networks were less involved with their patient representatives or were unclear who served on which bodies.

Respondents reported selecting patients to participate in PCORnet governing bodies in a number of different ways. Most frequently, patients volunteered to serve, and there was an attempt to match patients to their interests. Some PPRNs highlighted that some of their patient representatives had backgrounds that helped them with their roles on governing bodies (e.g., they

¹⁰⁵ Interviews with patient engagement leads in each CDRN and PPRN indicated that patients served on the following task forces: the Ethics and Regulatory Task Force, the Data Privacy Task Force, the Patient-Reported Outcomes Task Force, the Patient and Consumer Engagement Task Force, the Rare Disease Task Force, and the Obesity Task Force.

were physicians, data security experts, researchers, etc.). Another frequently reported approach was that patients already serving in leadership roles at CDRNs and PPRNs were asked to additionally serve on PCORnet governing bodies.

A team comprising representatives of PCORI, the Coordinating Center (including the Patient and Consumer Engagement Task Force), and the Steering Committee's patient representative oversaw the formation of the Patient Council. To select patients to serve on the Patient Council, the team issued a call to CDRNs and PPRNs to nominate a single representative from their networks. CDRN and PPRN leaders either issued a call for volunteers or approached individual patients. The team leading this effort formed a group of application reviewers, developed a rubric to score the applications, interviewed the candidates over the phone, and ultimately selected four CDRN/PPRN representatives for the Patient Council. Other Patient Council members were invited to participate based on their leadership roles in patient engagement at PCORI or within PCORnet.

Patients' Experience Serving in PCORnet-Level Governance Roles

Respondents reported mixed experiences serving on PCORnet governing bodies. Some PPRNs reported that their patient representatives had positive experiences and felt that their voices were being heard. Other respondents reported that it was challenging for patient representatives to engage at the same level as other representatives because of a lack of experience or knowledge. One respondent described the patient experience as mostly observing meetings and occasionally assenting or consenting to decisions, rather than guiding or driving decisions.

Some respondents reported that their patient representatives requested assistance with their roles and responsibilities serving on PCORnet governing bodies and that, for the most part, the CDRNs and PPRNs themselves provided the assistance. In some cases, CDRNs and PPRNs proactively worked with patient representatives so that they felt prepared prior to PCORnet governing body meetings. Few respondents reported that patients sought assistance from PCORI.

Some CDRN and PPRN respondents could not identify specific contributions patients made to PCORnet governance or PCORnet in general. Some respondents were not sure which participants at task force meetings were patients, which some considered to be evidence of equity in representation between patients and other stakeholders. A few went so far as to assume that most or all PPRN representatives were patients or caregivers. Other respondents thought that patients contributed by representing their patient communities well on PCORnet governing bodies and possibly changing the perspectives of researchers and clinicians with regard to how they work with patients on research. A few respondents thought that patients' involvement in decisionmaking on research topics was notable. Other respondents questioned whether patients had made any substantive contribution to PCORnet governance because, from their perspective, patients had not been meaningfully integrated into the PCORnet governance model throughout most of Phase I.

Participants' Experience Serving on the PCORnet Patient Council

Patient Council members reported that during the early phase of the Council's existence, they invested a great deal of time trying to establish what their roles were supposed to be and to educate themselves about PCORnet issues (e.g., data privacy, consent, ethics, etc.) so that they could make informed policy decisions. The Patient Council chairperson was described as playing a key role in orienting the other members to the Patient Council roles because she was more experienced with PCORnet.¹⁰⁶ The chairperson also led efforts to develop the Patient Council's charter. Education of Patient Council members on PCORnet issues included bringing in approximately nine experts to speak about relevant topics, including members of the Privacy Rights Foundation, task force leaders, and the Coordinating Center PIs.

Despite their key role reviewing draft policies on behalf of patients, the Patient Council reported that their comments were not routinely incorporated into the revised policies. This led some members of the Patient Council to perceive that the policymaking process was not as transparent as it could be. To address this issue, they began to work with PCORI staff, who shared earlier versions of policy drafts with the Patient Council. The Patient Council would give their comments on these early drafts directly to PCORI staff. In addition, the council implemented an activity they named the "warm hand-off," in which they invited task force members overseeing policy drafting for a phone call to discuss the Patient Council's comments on those policies.

Patient Council members shared some examples of input they provided on task force policies that, if accepted, they believed would protect patients' interests. For example, the Patient Council asked that patient data not be sold to pharmaceutical companies or used for marketing purposes. In addition, they held the view that PCORnet's patient data should not be used to conduct cost-effectiveness research because its results could be used against patients' interests.

Engaging Patients in CDRN/PPRN-Level Governance

All CDRNs and PPRNs established one or more governing or advisory bodies within their local networks that included patient representation. Some CDRNs and PPRNs had advisory bodies organized by area (e.g., research, communications, patient engagement, etc.) in addition to a governance body. In some cases, networks established separate boards comprised only of patients. In this section, we report the ways patients were selected to serve in these roles (a potentially key factor in the success of patient engagement), the challenges patients faced, and patients' overall experience serving in their roles.

¹⁰⁶ The Patient Council chairperson asked PCORI to establish the Patient Council as an additional way to engage patients in PCORnet policy development.

Approach for Engaging Patients in CDRN/PPRN-Level Governance

Selection of patients to serve in governance roles. Many CDRNs recruited patients to serve on their governing or advisory bodies by asking clinicians, researchers, or other patients in their network to recommend or directly recruit patients. CDRNs typically used their own internal networks of patients and family members to identify rare-disease patient representatives. CDRNs also reported recruiting patient representatives from community organizations.

Many patient representatives were already known to network leaders because of their roles establishing patient advocacy organizations, because they had worked together on past projects, or because they were already serving on another advisory board. Some networks preferred to work with patient representatives that had been involved with advocacy or had served in a professional capacity previously because they tended to be more knowledgeable about research than patients without advocacy backgrounds.

Many PPRNs existed previously as affiliated patient advocacy groups, so the assignment of patients to governance and advisory boards was a natural process that built on existing patient leadership in those groups. A few PPRNs had a pool of patient candidates to choose from because they administer patient representative training programs that teach patients about research. In a few cases, PPRNs asked their staff (e.g., patients, researchers, or clinicians) to directly recruit patients that they knew. In those cases, some people considered patients' backgrounds and skills that might be useful to have at the PPRN, such as having a legal or clinical background. In other cases, they recommended patients who they thought would be active participants or who would be "seen as authoritative . . . or trusted voices."

For PPRNs, patient representatives' ability to reach out to patient advocacy groups was a prevalent theme in the reported strategies for placement of patients on internal governance and advisory boards. Many PPRNs also used a nomination process through social media or organizational networks seeking applications and then interviewed candidates or asked patient communities to vote on candidates.

CDRNs and PPRNs expressed a range of perspectives regarding the attributes of "ideal" patient representatives. Some networks sought patients who were "educated," "motivated," and "interested in research," while others looked for "ordinary" patients. However, in many cases, respondents were unable to comment on the specific criteria used to select patients.

Roles of patients. Patient engagement leaders reported that patients contributed to CDRN and PPRN governance in many different ways. At many PPRNs, patients developed content or reviewed content that was presented on PPRN portals and websites, provided input on platform capabilities, and gave feedback on marketing materials. They worked with investigators to design surveys and review and test survey questions. In terms of research, respondents reported that patients in governance helped select or prioritize studies, helped decide on guidelines for selecting studies, and gave feedback to researchers on the treatment of patients in research studies. Patients also were reported to play an active role recruiting other patients. PPRN

respondents (and some CDRN respondents) also reported that patients participated in policy development and reviewing data use requests. If patients had additional skill sets that could help with PPRN decisionmaking (e.g., legal, research, technology), those skills were often leveraged in the PPRN. A few respondents provided examples in which patients actively challenged clinicians and researchers about their opinions, and respondents expressed an appreciation for that kind of input.

CDRN respondents reported that patients were involved in the development of mostly patient-related policies, provided input on potential research questions, participated in the development of their cohort survey, and reviewed data use requests. A few CDRNs reported challenges in engaging patients in these and other governance activities because those activities were described as being “less interesting” to patients.

Patients’ roles and level of representation in governance seemed to differ between CDRNs and PPRNs. Many PPRN respondents reported that their governing bodies were designed to have patients as an equal or majority voice, and, to achieve that, numerous patients had to be involved at every level of the network. The level of patient representation varied more across CDRNs. A few CDRNs reported having only one or two patients providing routine input on all governance issues, but most CDRNs engaged multiple patients to serve on all governing and advisory boards. These findings are broadly consistent with other health information technology infrastructure projects of national scope in which patient engagement activities received lower priority in the projects’ initial years, when core infrastructure was being established.¹⁰⁷

PPRN respondents also frequently discussed the balance in representation between patients and nonpatients on governing and advisory bodies. Respondents emphasized the equal standing that patients and nonpatients had on PPRN governance issues. In some cases, patients served as chairs or co-chairs of the PPRN’s primary governance body. In other cases, PPRNs set aside half or more than half of governance body seats for patients.

Challenges engaging patients. All of the CDRN patient representatives identified challenges understanding the research process for the diseases on which their CDRN focused, as well as the roles that individual CDRNs, PPRNs, and PCORnet as a whole played in research. Some CDRN patient representatives noted that technical language was one of the biggest barriers to participation, and they stated that they found it challenging to keep up with discussions during both their CDRN internal meetings and the PCORnet Patient Day retreat.¹⁰⁸ In contrast, PPRN patient representatives reported either not experiencing any challenges or simply being challenged by the work itself (e.g., timelines, difficulties getting their submissions through IRB, etc.), but not by their roles as patient representatives per se.

¹⁰⁷ Dullabh et al., 2012.

¹⁰⁸ The PCORnet Patient Day retreat was an opportunity for patient representatives to come together to discuss their experience participating in PCORnet. The Patient Day retreat was held during the winter of 2015.

Solutions pursued to address these challenges included developing a glossary for key terms, seeking support from mentors, and taking classes to better understand research. Specific topics mentioned as being challenging to follow were PCORnet’s data infrastructure and research methodology. At the same time, patient engagement leaders took steps to improve patient contributions, such as sharing materials for discussion with patients a few days prior to meetings so that patients have time to review them and having a process in which they check in with patient representatives routinely during meetings to answer their questions and obtain their input. A few CDRNs and PPRNs also addressed these issues by setting up special liaisons with whom patients could confer when they had questions during the course of their work.

No CDRN or PPRN patient representatives reported receiving any training or preparation for their roles. However, about half of the patient representatives did report receiving mentorship from network staff that helped them feel more confident in carrying out their roles. Many reported undergoing “on-the-job” training, in which their understanding of what the CDRNs or PPRNs were doing increased over time as they continued to attend meetings. Some patients endorsed the idea of undergoing formal training and hoped it would be something that all networks would consider supporting in the future.

In summary, patient representatives played an important role serving in local governance roles during Phase I. CDRNs and PPRNs drew on multiple resources to identify patients and appeared to vary in the use of specific criteria to select patients to serve in these roles. Patients developed and reviewed content destined for websites, patient portals, and surveys; prioritized topics for studies; and contributed to some aspects of study design. PPRN patients appeared to allocate a larger share of governance responsibilities to patients, which allowed patients to have equal weight as nonpatients in local decisionmaking within many PPRNs.

Patient representatives experienced challenges serving in their governance roles, including inadequate understanding of their role and lack of familiarity with language and technical aspects of research, and PCORnet’s distributed research network in particular. Patient representatives and patient engagement leads developed strategies on an ad hoc basis to help patients better perform their roles, although many indicated that formal training would be helpful in the future. Patients’ understanding of their roles evolved over time, although PPRNs appeared to demonstrate a stronger commitment to patients’ roles from the beginning of Phase I. Perhaps as a result, PPRN patient representatives were more likely to report making specific contributions to their networks.

Engaging Clinicians

Engaging clinicians was a priority for both PCORnet leadership and individual CDRNs and PPRNs. According to all groups, clinician participation was considered vital to the development and sustainability of the network because of clinicians’ unique role at the interface between research and patient care: Clinicians are not only positioned to offer their clinics as research sites

or enroll patients in studies, but they can also contribute valuable insights into research questions and support effective data collection strategies. By using PCORnet to answer research questions, clinicians might also contribute to PCORnet's sustainability.

Approach for Engaging Clinicians

PCORnet-level strategies. Some members of PCORnet's leadership reported that the network did not have a clearly defined plan for how to involve clinicians in the development and sustainability of PCORnet during Phase I. However, respondents noted that a few different strategies were either considered or implemented. One respondent reported that PCORI had briefly considered the development of clinician surveys about their areas of interest as a way to begin integrating clinicians into the PCORnet research paradigm, but this approach was not pursued further. Another respondent pointed to PCORI's efforts to encourage CDRNs and PPRNs to develop mentorship and training programs as a way to engage clinicians. In general, respondents reported that most of the responsibility for clinician engagement fell to individual CDRNs and PPRNs, whose contractual requirements included efforts to engage clinicians.

CDRN/PPRN-level strategies. To meet their contractual requirements, CDRNs and PPRNs deployed a range of strategies for engaging clinicians in PCORnet research. Techniques used by individual networks included distributing information to clinicians, discussing research priorities, providing incentives for participation, leveraging or expanding other collaborations (e.g., data-sharing agreements or research partnerships), "selling" PCORnet's value proposition, and working to align research and clinical workflows. Each of these is described below. While nearly all respondents reported using at least one strategy to reach out to clinicians, one CDRN reported that it had not attempted to engage clinicians because trials had not yet commenced, and, thus, it was "too early" to meaningfully engage clinicians.

Information-sharing. Several PPRNs reported strong communication channels with clinicians dating from the period preceding Phase I. A few networks described their dissemination of professional newsletters as a way to maintain contact with clinicians who specialized in their conditions of interest. Other PPRNs described hosting annual face-to-face meetings with clinicians on the topic of patient engagement or holding webinars to educate clinicians on their rare conditions—both of which were seen as successful. CDRNs were less likely to describe strategies involving information-sharing but, rather, focused their efforts on identifying information gaps and setting research priorities in conjunction with clinicians in their network.

Incentives. A few networks described incentives that were successful in attracting clinicians to participate in their network. One PPRN described how clinicians could benefit from a possible forthcoming pragmatic trial, could receive free grand rounds and continuing medical education credits, could participate in a PCORnet clinician-focused blog, and could gain access to other resources, such as free videos that could assist with diagnosing the PPRN's condition(s). Another PPRN offered clinicians access to a major clinical database, and, in an effort to establish an

online presence, the PPRN promoted the ability to engage communities of clinicians online and to provide a customized landing page that helped promote the clinician’s work or clinic within the online community.

Collaborations. A few CDRN respondents reported that their primary mechanism for engaging clinicians involved collaborations at the health system level or collaborations with other PPRNs. One CDRN described a two-part strategy for “selling” PCORnet to clinicians by first talking with the medical director or chief medical officer and emphasizing why PCORnet would be valuable to both patients and the health system. Then the CDRN’s engagement director met with clinicians “on the ground,” where the CDRN discussed how it functions operationally. Another approach to engaging clinicians taken by one CDRN was to better align clinical research and patient care by enhancing the functionality and interoperability of EHR systems at community practices and hospitals within the CDRN so that clinical data could be collected and shared.

Challenges Engaging Clinicians

The most frequently reported barriers to engaging clinicians in PCORnet activities were related to the limited role of clinicians in many networks’ recruitment plans, perceptions by clinicians that PCORnet represented “competition,” and time constraints or scheduling issues. These findings echo those of other large-scale health information technology infrastructure projects, which identified similar challenges engaging clinicians, mostly due to cultural resistance and competing priorities.¹⁰⁹

Recruitment plans excluded clinicians. Many of the PPRN respondents commented that the way in which they recruited patients into their networks was the primary factor limiting their engagement with clinicians. For example, PPRNs’ recruitment plans simply did not rely on clinics to enroll patients in the network, and many patients may have enrolled in a PPRN without the knowledge of their doctors. As a result, the incentive to perform initial outreach to clinicians was limited in several networks.

Perception of competitive threat. CDRNs and PPRNs that actively engaged clinicians occasionally encountered clinician resistance to participating in network activities. Some clinicians perceived that the new networks would disrupt existing relationships built around clinical research, that they would have to compete with CDRNs and PPRNs to enroll patients in studies, and that the networks would be collecting data to evaluate clinicians’ practice patterns and patient outcomes. Among these challenges, the most threatening from the perspective of clinicians (and one that was consistently reported by both CDRNs and PPRNs) was the potential for PCORnet to disrupt existing arrangements between the pharmaceutical industry and academic medical centers. According to one CDRN respondent, industry had been purchasing

¹⁰⁹ NORC at the University of Chicago, 2015.

data from one of its health systems, but PCORnet might emerge as another supplier of data, which would threaten a key source of revenue.

Other barriers. CDRNs often cited difficulties having to work around clinicians' busy schedules as another barrier, and some CDRNs concluded that, for some providers, participation is simply viewed as not cost-effective. In general, CDRNs had more difficulty than did PPRNs in convincing clinicians of the value that PCORnet could add to their practice.

Overall, respondents suggested that the most critical factor to securing clinician engagement is the successful implementation of the network. Until PCORnet is able to demonstrate its value to clinicians as a functional and useful network, CDRNs and PPRNs expect to struggle with achieving broad-based buy-in for the network.

Facilitators of Clinician Engagement

Factors that facilitated CDRN and PPRNs' engagement with clinicians included efforts to learn what types of incentives were important to clinicians, defining specific roles for clinicians, and strengthening or expanding clinician relationships that were in place prior to Phase I. Other less commonly reported factors included networks' ability to leverage previous work with particular organizations or on certain topics, PCORnet's patient-centeredness focus, and PCORnet's ability to help clinicians navigate regulatory demands of research.

Identifying appropriate participation incentives. In order to facilitate clinician engagement, many respondents began by simply listening to clinicians in order to learn how to best incentivize their participation in PCORnet. For example, to learn about the hurdles clinicians faced entering patients' data into its disease registry, one PPRN polled clinicians who had and had not regularly entered data into its registry. The PPRN learned that the best incentives for engaging clinicians were financial incentives and recognition on the network's website. Other reported incentives included providing clinicians with access to websites where they could learn more about caring for patients with certain conditions, as well as access to other specialists who could provide second opinions. Similarly, a CDRN polled clinicians and learned that clinicians most valued webinars because they could exchange perspectives and interact with other webinar participants. The least preferred type of communication was attending face-to-face meetings because of the amount of time and travel required.

Defining clear roles for clinicians. About half of respondents felt that clinicians were most effectively engaged by defining clear roles for them within their network's research program. Many PPRNs discussed specific roles for clinicians in their networks. PPRNs appointed clinicians to serve as their network's scientific director and, in at least three cases, as the co-PI. Many PPRNs reported that clinicians were given key roles helping to recruit patients, serving on network advisory boards, or participating in patient outreach groups.

Several CDRNs also established specific roles for clinicians in their networks. For example, one CDRN created an infrastructure comprising the senior scientific leaders at each of the institutions within their network that allowed the CDRN to engage individual clinicians at each

of the institutions by forming working groups around specific issues. Another CDRN created a clinical oversight group comprising clinicians from across all of the CDRN's partnering health systems and also created a clinician-staffed committee that focused specifically on EHR issues.

Leveraging preexisting relationships. Some PPRNs indicated that their previous relationships were critical to successfully engaging clinicians in PCORnet activities. Two PPRNs reported that they leveraged clinician networks and partnerships that they had cultivated for at least 16 to 20 years. Such relationships were commonly reported by rare-disease PPRNs, whose clinician partners tend to be familiar with one another. One PPRN collaborated with genetic counselors and academic institutions for many years before PCORnet and has used its connections to recruit patients into trials and disseminate research results.

Other facilitators. CDRNs and PPRNs reported assorted other facilitators of clinician engagement. According to one PPRN, focusing on patient-centeredness has also attracted the attention of clinicians and has helped to overcome competitive barriers between academic institutions collaborating with the PPRN. In addition, one respondent noted that tailored communication strategies to clinicians have been effective.

In summary, while widely regarded as critical to PCORnet's sustainability, the responsibility for engaging clinicians fell almost exclusively to CDRNs and PPRNs that used a range of strategies to do so, including providing information, tools, and resources to clinicians in return for their participation; discussing research priorities; leveraging or expanding other collaborations; "selling" PCORnet's value proposition; and working to align research and clinical workflows.

CDRNs and PPRNs faced challenges engaging clinicians, including limited time, the limited role of clinicians in many networks' patient recruitment plans, and perceptions by clinicians that PCORnet represented "competition" that might disrupt existing research networks or revenue streams. Facilitators included taking steps to learn what types of incentives were important to clinicians, defining specific roles for clinicians, and strengthening or expanding clinician relationships that were in place prior to Phase I.

While it is difficult to assess the intensity or outcomes of clinician engagement, as a whole, clinician engagement appears less robust than that of other stakeholder groups. Until PCORnet is able to demonstrate its value to clinicians as a functional and useful network through demonstration projects, CDRNs and PPRNs might struggle with achieving broad-based buy-in for the network.

Engaging Health System Leaders

Engaging health system leaders was viewed as a priority for CDRNs for whom continued support for their operations would be critical to ensure their sustainability after Phase I, when PCORI's level of funding to CDRNs was scheduled to drop to "infrastructure maintenance" levels. For all participants, health systems play a critical role providing data to support

collaborations between CDRNs and PPRNs and for PCORnet-wide research projects—particularly for supporting infrastructure changes that would support the integration of clinical research and patient care.

Approach for Engaging Health System Leaders

PCORnet-level strategies. PCORnet leadership identified a few key opportunities to engage health system leaders during Phase I. First and foremost, PCORI convened an Institute of Medicine (IOM) workshop involving numerous health system CEOs in April 2014 titled “Health System Leaders Working Toward High-Value Care Through Integration of Care and Research.” Respondents felt that this meeting was a productive one that not only helped PCORI and PCORnet to establish new contacts with health system executives but also served as a force multiplier for existing efforts to address the needs and interests of PCORnet stakeholders. As one respondent stated, “suddenly you’ve got health plans talking to the leaders of these huge systems with which they do a huge amount of business about common concerns and interests.” In general, most of PCORnet’s leaders believed that the goodwill fostered as a result of this meeting was a contributing factor to their perceived success in engaging with health systems. The Health Systems Interactions and Sustainability Task Force extended these efforts by identifying new opportunities for health system involvement and for developing a framework to sustain those relationships.

In addition to the IOM meeting, PCORnet leaders also utilized more informal methods of engaging with health system leaders. For example, some leaders used their existing relationships with integrated health systems to foster research partnerships and increase the overall visibility of the PCORnet initiative. Most importantly, however, the majority of respondents reported that the most successful strategy for engaging with health systems was to describe the potential value of PCORnet to their work, which was identified as building a national system that facilitates data analysis and comparisons of patient populations within and across health systems. For business-oriented entities, such as health systems seeking to provide low-cost, high-quality patient care, this resource was thought to be especially valuable.

Health system demonstration projects represent another strategy used by PCORnet leadership to engage health systems. These demonstration projects would accomplish three aims: (1) to identify questions of high priority to health system leaders that can be answered using the common data model,¹¹⁰ (2) to familiarize health system leaders with the PCORnet model of using data to ask and answer questions of interest to health systems, and (3) to demonstrate the value of PCORnet investment for health system leaders.

CDRN/PPRN-level strategies. The strategies used by CDRNs and PPRNs to engage health system leaders relied primarily on existing relationships and the articulation of an attractive

¹¹⁰ Dupont et al., 2009.

value proposition for each contact. Nearly all CDRNs reported that PCORnet helps health systems to advance their various missions by conducting research on topics of relevance to their patient populations, supporting the concept of the “learning” health system, benchmarking health system performance, and enhancing clinician engagement to improve decisionmaking and patient outcomes. A few CDRN respondents felt that participating in PCORnet added prestige to their institutions because of the public service mission of PCORI and the positive implications of collaboration for an organization’s reputation.

Given the preliminary nature of PCORnet’s research portfolio and capability during Phase I, CDRNs and PPRNs had to identify several dimensions of PCORnet value for health systems in order to facilitate constructive engagement relationships. The most successful value propositions described by these networks include the following:

- **Better, faster, cheaper research.** CDRNs overwhelmingly described PCORnet’s value to health system leaders in terms of offering a new infrastructure that could support better, faster, and cheaper research on topics of interest to the health system. About half of respondents suggested that PCORnet’s ability to help answer research questions that facilitated decisionmaking between clinicians and patients would help both networks and health systems to achieve their missions.
- **Access to data to inform quality improvement and manage population health.** About half of CDRN respondents discussed the advantages to health systems of having a common infrastructure for sharing aggregate data to strengthen their research programs, enhance population health management, and promote practice improvement. In general, health systems participating in CDRNs expressed considerable interest in using PCORnet’s clinically rich, curated patient data to support these activities.
- **Benchmarking quality and value.** Several respondents highlighted PCORnet’s ability to support “faster and more useful improvements” in quality reporting, using PCORnet data as a strong selling point to health system leaders. In particular, respondents noted that health system leaders attached significant value to PCORnet’s potential role in allowing comparisons of performance between health systems. Some respondents noted that the overall value of the benchmarking exercise is an integral part of the “learning health system,” which is a model for continuous quality improvement at the systems level. According to respondents, using PCORnet data in this manner could facilitate collaborations with higher-performing clinics within the network. Some CDRNs reported that health systems are interested in using PCORnet’s benchmarking capabilities as part of a larger strategy for pursuing accountable care organization contracts.
- **Enhancing collaborations.** For about half of respondents, PCORnet has helped to enhance existing collaborations centered on health systems’ data infrastructure. For example, one CDRN respondent noted that its network had already been working

with health plans in different regions to put data into a central repository. Other CDRNs noted that participating with PCORnet would allow clinics to participate in collaborations with other leading research centers, such as those participating in patient safety networks. Smaller health systems highlighted the benefits of collaboration in terms of gaining access to innovations and resources that would not otherwise be available to them.

Engaging Health System Leaders: Summary

CDRNs have worked to articulate PCORnet's value proposition for health systems and have conducted outreach to health system leaders. Despite interest in PCORnet's ability to deliver highly curated data to facilitate quality improvement, benchmarking, and managing population health, we were unable to elicit perspectives of health system leaders directly to better understand their willingness to support PCORnet through continued or enhanced funding beyond Phase I. Most of the information reported on health system engagement comes from interviews with CDRN and PPRN PIs.

Nevertheless, PCORnet has laid the groundwork for closer engagement between CDRNs and health system leaders during Phase II by convening an IOM meeting of leading health system executives and mobilizing additional funding to support greater engagement with health system leaders. This will ultimately lead to the development of topics that are considered high priority by health system leaders and that can be studied feasibly within PCORnet. Prospects are high for closer engagement and the development of a more coherent value proposition for health systems during Phase II.

Engaging Potential Federal Funders and Users of PCORnet

PCORnet maintained relationships with a variety of federal partners over the course of Phase I. Federal partners' primary involvement in the network was to ensure, to the extent possible, that the PCORnet data and research infrastructure could meet or extend organizational goals. In addition, as several partners are also funders of research, engaging these partners early was viewed as a way to address early on any potential concerns funders might have with future research studies conducted within PCORnet.

Engaging Federal Partners

Federal partners engaged with PCORnet largely by assigning a representative who participated on the Steering Committee or attended Coordinating Center meetings. These representatives varied in their level of engagement and rates of attendance at meetings. When applicable, some agency representatives contributed specific expertise, such as on the development of the common data model and on standards for a Unique Device Identifier.

Federal partner engagement in the first phase of PCORnet consisted mostly of agency representatives becoming involved in regularly scheduled meetings (e.g., Steering Committee meetings). Respondents who served in these roles reported varying levels of engagement that also seemed to change over time. While some respondents reported active involvement or the belief that their thoughts were listened and responded to, other agency representatives were much less involved and/or became less involved over the course of Phase I. The degree to which PCORnet's direction and activities were perceived as relevant to the needs and mission of an agency seemed to affect the level of engagement of the agency representatives.

Challenges Engaging Federal Partners

When queried on how satisfied federal partners were with their involvement in PCORnet, most seemed generally satisfied, with a few being more or less enthusiastic about their experience. Representatives from three agencies stated that they were satisfied with their level of involvement, and one elaborated that "there are a number of places [within PCORnet] where the federal voices are being heard." Other agencies seemed satisfied with their involvement but still felt that their agency's priorities were not shared by PCORnet. For example, one respondent was disappointed that, despite their early involvement, PCORnet was not moving in a direction that would meet the needs of the agency.

In describing their involvement, agency representatives commented on the way that PCORnet involved federal partners. One respondent described PCORnet's governance and policy-setting process as having too many layers and being cumbersome. This type of overly complex process likely made partners' involvement with PCORnet more tedious or burdensome than necessary. Another representative thought that PCORnet had been a "bit too internally focused, not reaching out to external stakeholders." As evidence, this respondent described how there are no "external people" on the Executive Committee, which, as a collaborator, was discouraging to see. This respondent further criticized PCORnet's governance as being so process oriented that it interfered with its ability to take strategic action.

PCORnet's attempts to engage and coordinate with so many stakeholders, including but not limited to federal agencies, resulted in moderate levels of engagement, with some respondents expressing frustration about some processes. Although establishing governance procedures was necessary to the start-up phase, the level of bureaucracy required to manage the multiple partners and parallel processes within PCORnet has led to frustrations that some partners suggest were inhibiting progress. PCORnet's evolving governance model may address some of these concerns. As part of the new model, both federal and industry partners will form a new PCORnet Advisory Group that will meet independently of the Steering Committee, will set priorities and agendas in collaboration with the PCORnet Executive Committee, and will interact with the Executive Committee on a quarterly basis.

Federal Partner Perceptions of Challenges to Be Addressed

Respondents from federal agency partners raised a wide range of concerns when asked about the challenges that PCORnet needed to address. Governance was a central theme, as were data issues, sustainability, and the ability of the network to meet the needs of all stakeholders. To a lesser extent, respondents voiced concerns about ethics and the flexibility of the network to adapt to changes, as discussed below.

Governance challenges. Most respondents from federal agencies cited the governance of such large endeavors as PCORnet as an inevitable challenge. General coordination and messaging was described as difficult; the effort involved with “getting everyone on the same page” with such a large group of stakeholders was not underestimated. Determining policies and procedures for research procedures, including data access and how studies would be selected or prioritized—especially in the case of investments from multiple parties with conflicting interests—were two other notable governance challenges that were of concern to federal partners.

A few respondents highlighted strategic decisionmaking about overall direction or evolution as a challenge going forward. Federal partners acknowledged that there was a tension between meeting the needs of all stakeholders and having strong or deep enough capacities to adequately meet the needs of users. Currently, some respondents felt that there were so many stakeholders that the system had not developed enough capabilities to meet anyone’s needs. A related tension has been between the public interest and the grantee or other stakeholder interests. Centralized leadership with more proscriptive policies could have staked out a clearer sense of public interest, rather than allowing grantees so much control over the formation of the network, which fostered engagement and buy-in but may have clouded the vision of PCORnet being for the benefit of patients.

Data challenges. The second major challenge to PCORnet’s forward progress had to do with data. Respondents pointed to limitations of the common data model, technical issues related to data extraction and quality, and linking data from across so many networks’ data systems. One respondent cited the challenges of selecting the common data model and suggested that PCORnet should have exercised stronger leadership around choosing a data model, rather than trying to gain consensus around one as a group. This respondent speculated that a challenge related to the limitations of the common data model that PCORnet will face in the future is how to establish standards to enable incorporation of vast amounts of other clinical data, such as information from medical devices. This concern about expanding the common data model was echoed by other federal partners, who thought that the variables contained in the common data model were few relative to the number of observations it would eventually be spanning, leading to a “slim” dataset that had breadth (of observations) but little depth (adequate data elements to do meaningful research).

Other data concerns mirrored the agenda of some of PCORnet’s task forces, including unique identification of records, linking, and representativeness of data. One respondent reflected that the data challenge mirrors the “fundamentally heterogeneous and fragmented system” of health care delivery in this country, including the challenge of controlling or accounting for delivery system factors when looking at patient outcomes.

Sustainability challenges. Most respondents from federal agencies perceived that planning for future funding and ongoing sustainability was a looming challenge. One federal partner noted that the current capabilities and goals of PCORnet make the potential funders a fairly narrow group, and that expanding the relevance of PCORnet through increasing flexibility and access might attract more potential funders. Another respondent also mentioned the need to expand PCORnet’s capabilities, pointing out how limited the data coverage is at this point. Several partners mentioned ideas for sustaining PCORnet, including a combination of government and industry funding, but respondents also noted problems with the conflicting expectations and conventions of different types of funders (i.e., around access to data and intellectual property).

Other respondents felt that it would be helpful for PCORnet to focus part of its attention on assessing the financial and time costs of the PCORnet effort. A few respondents noted that there are too many tasks and that data collection efforts may be suffering. A related suggestion by one respondent was that PCORnet should consider better understanding clinical trial costs, including data collection costs and patient enrollment expenditures, for the PCORnet system, which could help support sustainability planning.

Ethics and regulatory challenges. A few respondents named ethics and regulatory issues as challenges facing PCORnet because of the network’s weighty responsibility for managing patient consent and protecting data received from partners across the country. One respondent cited challenges with obtaining IRB approval, especially relating to issues of consent for patient-level data. Another respondent noted how some PCORnet policies may pose a challenge to certain researchers because of conflicting policies at the organizational level, such as reimbursing patients, which is part of PCORnet policies but is not routinely done by NIH. One respondent raised the ethical challenge of how the results of research from the network might become a barrier to care if results are generalized inappropriately. This respondent worried that the hazard of developing such a powerful clinical research network might be that results are taken without the appropriate caveats necessary for any observational study.

Engaging Federal Stakeholders: Summary

Overall, most federal partners reported being satisfied with their governance roles during Phase I, although some representatives indicated a preference for more direct channels to provide input into PCORnet’s decisionmaking. Federal partners perceived many governance and data challenges for PCORnet as it moved into Phase II—particularly the network’s ability to make decisions efficiently that appropriately balanced stakeholders’ priorities. The limited breadth of PCORnet’s current data resources also raised some concerns, and federal partners expressed

hope that PCORnet would expand its data model, accommodate diverse funding streams, and develop better estimates of the cost of future research to support sustainability planning.

Despite these concerns, the view held by federal partners was that PCORnet was generally on the right track. Respondents indicated that they have seen PCORnet progress, as demonstrated by the network's continued evolution, which has been informed by stakeholder feedback. Other respondents also commented that it may be too soon to tell whether PCORnet is on the right path because the pilot studies are still unfolding and PCORnet's governance structure continues to evolve.

Engaging Potential Industry Funders and Users of PCORnet

PCORnet's pharmaceutical, medical device, and health insurance industry partners expressed varying levels of enthusiasm for the potential for PCORnet to meet the needs of their members. Industry stakeholders hoped that PCORnet could reduce the time required to conduct trials, enroll patients more quickly, and be more cost effective than the current model for clinical trials.

Approach for Engaging Industry Stakeholders in PCORnet

Specific industry groups' engagement with PCORnet varied but included participation in meetings of the Steering Committee, Coordinating Center, Governance and Collaboration Task Force, Health Systems Interaction and Sustainability Task Force, and quarterly ad hoc meetings with PCORI staff. Industry respondents offered examples of their participation, including, for example, making suggestions about data standardization policies.

PCORnet conducted outreach to the broader pharmaceutical, device, and diagnostics industry through a series of workshops in March 2015. Attendance at these workshops was strong and included senior industry staff, and it was reflective of a generally high level of interest in PCORnet from industry. Industry respondents reported that these attempts to reach out to industry were well received. A respondent from PCORI also spoke on the topic of industry engagement during an interview and cited strong interest and plans being developed for collaboration with two companies, and another company had expressed interest but, as of the time of the interview, had not made any formal agreement to collaborate with PCORnet.

Challenges Engaging Industry Stakeholders

While some stakeholders engaged regularly and were satisfied with their involvement, others felt that their involvement came too late in the course of the project and stated that they would have preferred to give even greater input into Phase I decisionmaking, which left these stakeholders feeling fairly dissatisfied with their experience. For example, one stakeholder with roots in the health insurance sector reflected on early missteps of convening partners, complaining that "the recognition that health plans needed to be part of this [PCORnet] probably came a little late, and probably could have been brought deeper into the discussions earlier."

Another respondent echoed this sentiment and urged more targeted involvement with industry moving into the next phase of the project so that a broader group of industry might be engaged, potentially as “stakeholder-funders.” A few respondents also noted that it was impractical having a single representative for such large and diverse sectors.

Industry Stakeholder Perceptions of Challenges to Be Addressed

When asked about major challenges for PCORnet, industry respondents focused on challenges related to the competing interests of multiple stakeholders. Major concerns were who drives research, who “owns” a study question, and how study prioritization would occur.

Governance challenges. Industry respondents were uncertain and concerned about the ability of PCORnet’s existing governance model and processes to meet the needs of industry. For example, if a company sponsored a research project, respondents questioned whether they would have access to the results in advance of the results being publicly available. Similarly, industry representatives were unsure whether PCORnet would be able to support the need for quick-turnaround responses on questions often required by biopharmaceutical companies or payers when a study is being developed. In comparison with the challenges cited by federal agencies, industry respondents focused more on the data governance and technical details, rather than the overall direction or goals of PCORnet, which were primary concerns for federal agencies.

Industry stakeholders also questioned the “bandwidth” of PCORnet for meeting the needs of all users and desired more clarity on the processes that PCORnet would use to work with so many different stakeholders. At this point, one respondent said, it is not clear how studies would be prioritized and selected, and whether PCORnet would be able to respond to the types of ad hoc requests—for example, generating additional analysis that was not specified in the original study—that industry routinely requires. Another respondent raised a concern about intellectual property and control of findings. The respondent acknowledged that PCORnet was a public resource but felt that industry would need some kind of tangible benefit—like early access to study results—in order for PCORnet funding to really be an attractive option.

Communication challenges. Respondents identified ways that PCORnet could be more effective at communicating and working with its member organizations. They noted concerns about the way that PCORnet has communicated with industry groups, and several respondents described specific risks or concerns held by industry to which they thought PCORnet should be responsive. Respondents wanted PCORnet to communicate more clearly about its plans and directions, including being more transparent about the process of selecting studies. One respondent conveyed that a few health insurers were confused and felt misled by early efforts of PCORnet to solicit study ideas. This respondent thought that current communication strategies included a lot of “talking past each other” and cautioned that PCORnet should do a better job of listening to health insurers.

Another communication challenge involved the posture of PCORnet members toward industry groups. One respondent discussed how researchers often assume that industry has

conflicts of interest and is “heavily biased,” which affects the partnership dynamic. On the other hand, this respondent also described the PCORnet member institutions as “some pretty heavy hitters” who are confident in their ability to do the research and are much more interested in funding from industry than in learning from the experience of industry.

Other challenges. Other challenges mentioned by a few respondents related to the identity and sustainability of the network. Because PCORnet will not be supported exclusively with either private funding or federal funding, it will likely experience the tensions of sitting between two worlds. One respondent described the costs of studies and the challenge of funding the work. Even with efficiency, this respondent felt that the size of the studies would still result in high costs that would have to be recovered elsewhere, whether from industry or federal sources.

Industry respondents also perceived significant risks associated with the use of evidence produced from a large clinical research infrastructure like PCORnet. A few respondents expressed concerns about the possibility that large, clinical efficacy trials run through PCORnet would lead to more homogeneous care patterns by providing weighty evidence in favor of one treatment to the exclusion of others. Respondents whose business depended on developing new treatments saw value in PCORnet of making sure that “a fair evaluation of performance” is conducted.

The counterpart to these serious questions about the capability of PCORnet to be a useful resource for the industry was what one respondent described as a “‘show me’ attitude” in which industry representatives are thinking, “‘Okay, prove it to me, show me what you can do. I’m willing to go this far. Show me what I value I can get out of it.’” In the context of this hesitant, “wait and see” attitude, the challenges enumerated by industry respondents may be less of a barrier to engagement than their desire to see the products of the demonstration projects or other results that prove PCORnet’s capabilities.

Engaging Industry Stakeholders: Summary

While PCORnet has engaged with industry stakeholders throughout Phase I, several stakeholders reported feeling that they were engaged too late, and several stakeholders have questions remaining about how research studies are initiated and conducted within PCORnet. Even respondents that affirmed the value of a large, integrated, research-ready clinical data network tended to be measured in their support for PCORnet, often noting that PCORnet remains in a formative stage. Quickly establishing PCORnet’s capabilities through demonstration projects appears critical to demonstrating value for future industry funders.

To support and be responsive to industry needs, PCORnet will need to devote more time to developing relationships with this group to understand their motivations and doubts and to effectively communicate the value proposition of partnership. In particular, the value of PCORnet to health insurers appeared least clear to some respondents and had not received much attention within PCORnet. To address these issues, PCORnet launched four initiatives near the end of Phase I.

First, PCORI's Board of Governors appropriated funding in the amount of \$6 million to support demonstration projects that specifically engaged health plans. These efforts could pave the way to broader participation from insurers, which would make the data in the network more complete and useful for all partners.

Second, to address some of the industry stakeholders' questions about how research studies are initiated in PCORnet, the Executive Committee initiated the Front Door Policy Work Group, which was tasked with developing a process for collecting, evaluating, and triaging research opportunities. By August 2015, the work group had developed initial guiding principles for the selection of opportunities, with a focus on systematic, transparent, efficient, and timely approaches. As Phase I drew to a close, the work group was working to develop the procedures and processes for opportunity selection and was preparing to share initial drafts of these policies with the Coordinating Center and Executive Committee.

Third, the new PCORnet Advisory Group created at the end of Phase I and comprising both federal and industry partners may help to improve engagement with these key stakeholder groups in Phase II. The Advisory Group will develop an agenda and interact with the Executive Committee on a regular basis, which may help to ensure that the priorities of these stakeholders are communicated to PCORnet's leadership in a more effective way than in Phase I.

Finally, PCORI has also made concerted efforts to continue building relationships with industry groups through the establishment of the Industry Collaboration Work Group. Starting in June 2015 with the selection of work group members, the work group has participated in various outreach activities with industry partners to better understand the concerns, research needs, and goals of industry in order to better integrate PCORnet into industry research workflows. Concurrent with these efforts, the work group has developed draft criteria for pilot studies that could be used to support the infrastructure for industry research projects, as well as initial guiding principles for industry collaboration. By December 2015, the work group seeks to select industry pilot projects and identify therapeutic areas or populations for future studies.

Stakeholder Engagement: Summary of Key Themes and Priorities for Phase II

CDRNs, PPRNs, and PCORnet leadership played different roles engaging patients, clinicians, and health system leaders during Phase I in the design, use, and sustainability of PCORnet. Industry and stakeholders also served in PCORnet governance roles and voiced several concerns that they hoped would be addressed as the network heads into Phase II.

CDRNs and PPRNs engaged patients to serve on local governance and advisory bodies, as well as in PCORnet-level governance roles. PPRN patients appeared to allocate a larger share of governance responsibilities to patients, which allowed patients and nonpatients to have equal weight in local decisionmaking within many PPRNs. While patient representatives experienced challenges serving in their governance roles, patient representatives and patient engagement

leads developed strategies on an ad hoc basis to help patients better perform their roles. PCORnet might consider formal training to facilitate patients' participation in the future that would help to ensure that the patient's voice can be heard in all PCORnet activities. PPRN patient representatives, in particular, reported making meaningful contributions to their networks.

Responsibility for engaging clinicians fell almost exclusively to CDRNs and PPRNs that used a range of strategies to involve clinicians in their network activities, including providing information, tools, and resources to clinicians in return for their participation; discussing research priorities; leveraging or expanding other collaborations; and working to align research and clinical workflows. While the intensity or outcomes of clinician engagement could not be assessed directly by the evaluation team, clinician engagement appeared to receive less attention than that of other stakeholders, most likely because of physicians' competing demands and the lack of tangible evidence of PCORnet's value. Successful completion of PCORnet's demonstration projects will be critical for demonstrating the network's value to clinicians.

CDRNs have worked to articulate PCORnet's value proposition for health systems and have conducted outreach to health system leaders. Despite reports of health systems' interest in PCORnet, we were unable to elicit perspectives of health system leaders directly to better understand their willingness to contribute sustainability funding to their local CDRNs. PCORnet has laid the groundwork for closer engagement with health system leaders during Phase II by convening an IOM meeting of leading health system executives and mobilizing additional funding to support greater engagement with health system leaders in advance of future demonstration projects.

Federal and industry partners expressed specific concerns about both their participation in Phase I and PCORnet's development. Industry partners (and to a lesser extent federal partners) felt that PCORnet may have been too inwardly focused during Phase I. Some felt that they were engaged too late or simply not enough. As a result, several potential funders have many unanswered questions about how industry-funded studies might work in PCORnet. Their questions focused on process issues, including turnaround time, the mechanics of protocol development, and dissemination policies. Expanding outreach with industry should be a high priority for Phase II and should build on the March 2015 industry work group meetings, which were the first real opportunity for a large number of stakeholders to learn about PCORnet and open a dialogue about their operational concerns. The work groups established by the Executive Committee near the end of Phase I may ultimately help to address the needs and concerns of these stakeholders.

Another concern is that the value proposition for investing in PCORnet remains ambiguous for too many potential funders. PCORnet has struggled to make a compelling case for health plans to participate in PCORnet—with both participants and health plans acknowledging the issue. Potential industry funders also worry that the size of PCORnet trials will necessarily make them costly. As a result, many industry groups have taken a "wait and see" approach. While the \$6 million in funding appropriated for health plan demonstration projects may help to provide

examples of high-value projects, PCORnet would be well served by devoting greater attention to developing use cases for specific end users.

Finally, the degree to which CDRNs and PPRNs will be able to fund operations during Phase II (when funding from PCORI resets to a lower level) remains unclear. Some networks (especially PPRNs) reported “losing money” on Phase I activities, and some participants felt that PCORnet funding was insufficient to support the level of collaboration that PCORI might have anticipated. Other participants noted that the supplemental funding that sites will receive for conducting the ADAPTABLE trial is inadequate. Demonstration projects will be needed to better understand the resource requirements associated with PCORnet studies and the amount of funding needed to guarantee the network’s sustainability.

All PCORnet participants recognize the need to demonstrate the value of the network to potential funders, and most have aggressively pursued sustainability funding. Designing and appropriating funding for several demonstration projects that could provide initial evidence to support future investments in PCORnet were key accomplishments during Phase I. The selection, design, and launch of the 20,000-patient ADAPTABLE trial, in particular, will serve as a compelling test case for funders. Similarly, both the health system and health plan demonstration projects could go a long way toward building the value proposition for health systems’ investments in PCORnet and health plans’ contributions of data to support future PCORnet research.

9. Conclusion

Interviews with stakeholders, reviews of CDRN and PPRN quarterly progress reports, and observations of meetings of governance entities paint a picture of a network that has made considerable progress during the past 18 months and shows promising signs that it is capable of undertaking multi-site CER in its next phase. Below we summarize the network's key accomplishments and the most pressing challenges as the network heads into its next phase.

In the domain of governance, PCORnet established a governance structure and policy development process that has evolved considerably over 18 months to correct initial deficiencies. These changes suggest that the governance structure is responsive to feedback and will take action to ensure that decisionmaking is timely. Participants are broadly supportive of these efforts, but the new governance model remains largely untested, and participants will continue to push for a decisionmaking process that is more participatory and has greater transparency than the initial model. PCORnet must also work toward developing a coherent research agenda for the network to guide research during Phase II and more clearly specifying key operational details, such as how research will be initiated and conducted within PCORnet.

PCORnet's data infrastructure-building activities have produced a common data model and a transparent process for expanding the model. By month 16, CDRNs and PPRNs had collectively standardized data on tens of millions of patients. Most CDRNs and many PPRNs have made progress setting up their DataMarts and responding to test queries, and CDRNs and PPRNs have come together to address numerous common data-related challenges. PCORnet's success may depend critically on CDRNs' ability to obtain complete, longitudinal patient data from payers, which has proceeded slowly during Phase I. Another potential concern is the quality of PCORnet data, although data quality assessments have not been completed on a sufficient scale to date to draw conclusions about data quality. Developing biobanking capabilities, which received low priority during Phase I, should be given greater attention in the coming months to capitalize on widespread interest on the topic within PCORnet. Other remaining questions include the speed with which PCORnet can expand the common data model to improve its utility and to attract sustainability funding and the extent to which networks will be able to participate in queries, trials, and observational research studies at the scale envisioned by PCORI during Phase II.

CDRNs have made considerable progress implementing multi-site IRB review models, although few protocols have actually been reviewed by these new systems and their potential efficiency gains are hard to estimate at this point in time. Most CDRNs appear to have developed the important foundational capability to screen patients for clinical trial eligibility using standardized data contained in their DataMarts, and PPRNs have developed, tested, and deployed a wide range of outreach methods to identify and recruit members of their target populations into their networks. CDRNs may face challenges moving to a PCORnet-wide streamlined IRB model,

which is expected to be introduced during Phase II. Some CDRNs and PPRNs have encountered enrollment challenges and may not be able to initiate research on individual condition cohorts until well into Phase II.

Participants appear to have established a collaborative culture during Phase I. While the level of collaboration between CDRNs and PPRNs remains a concern heading into Phase II, CDRNs' Phase II requirements (which obligate them to partner with PPRNs) may help to strengthen partnerships in the future. Furthermore, while many PPRNs viewed their role as “secondary” during Phase I, greater coordination of PPRN activities within the Coordinating Center and the mobilization of additional PCORI funding to support PPRN-focused demonstration projects suggests that many new opportunities may be present for PPRNs to play a larger role in Phase II.

Patient and stakeholder engagement was generally strong during Phase I. Most networks have pursued sustainability funding primarily by engaging their local health systems and other partners in research-related collaborations, but the completion of multiple demonstration projects may be needed to provide a compelling case for funders to invest in PCORnet. Continued and enhanced engagement of industry and federal partners may be useful at the PCORnet level to ensure that stakeholder questions and concerns are addressed in a timely way.

Examining the full set of network activities, CDRNs and PPRNs have differed in their progress in ways that might have been expected, given their relative strengths. CDRNs have progressed farther in terms of data standardization, owing to priority accorded to them by the Coordinating Center. Meanwhile, patient engagement appears to be more robust among PPRNs. On issues of governance, CDRNs and PPRNs share a common view about the limitations of the early governance model and the limited role in decisionmaking played by networks vis-à-vis PCORI and the Coordinating Center.

Another cross-cutting finding was that participants valued opportunities for collaboration around technical issues that could leverage the collective expertise of the network to provide solutions for a wide range of implementation issues. For example, collaborations around developing or refining strategies for IRB streamlining, informed consent, data standardization, and data privacy were reported as being extremely helpful or in greater need. Moreover, across these various areas, CDRNs and PPRNs repeatedly cited the need for additional demonstration projects to advance work in these areas.

As PCORnet's infrastructure-building phase transitions into a new phase characterized by high levels of research activity, its evaluation needs will change significantly. For Phase II, the external evaluation should focus on the effectiveness of the PCORnet governance model in navigating the complexities of a vast research enterprise, including its ability to attract external funding, develop SOPs for prioritizing and initiating research, and establish a coherent research agenda that remains responsive to the priorities of both CDRN and PPRN participants and external researchers and funders. The evaluation should also shift its focus to research production by examining the volume, patient-centeredness, and potential impact of the research that PCORnet produces. As Phase II will feature the completion of multiple research studies, the

evaluation should also focus on the extent and manner in which PCORnet disseminates its research findings, including the potential impact on health care delivery within its constituent health systems.

Phase II provides an opportunity for the network to be guided by and evaluated on the basis of objective metrics of performance. In particular, Phase II metrics should enable an assessment of the degree to which PCORnet can impact the speed and cost of research, as well as its burden on patient participants and the health systems in which the research will take place. Phase II also allows opportunities to conduct a robust assessment of patient engagement that can ensure that PCORnet research is informed by patient priorities and preferences, regardless of the specific design or clinical focus of each study. Consistent with stakeholder-driven research, the evaluation should develop metrics, in conjunction with the PCORnet leadership, to leverage the clinical, informatics, and evaluation expertise of PCORnet participants.

In conclusion, in the face of high expectations, PCORnet achieved much during its brief initial phase of development. Despite the fact that key infrastructure for the network was put in place, many challenges remain. The degree to which PCORnet can scale up quickly in Phase II by expanding the common data model and launching studies that are supported by high-quality data and present compelling use cases to potential funders will determine the network's future success. Additionally, opportunities exist for a more robust and rigorous evaluation in Phase II—including the collection of quantitative metrics of performance—which can help PCORnet meaningfully track its progress over time and can also be used to support future external evaluation efforts.

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Appendix: Task Forces

Biorepository Task Force. The goal of the Biorepository Task force is to “develop and implement systematic approaches that support a regulatory-compliant, comprehensive, and sustainable Network-wide biorepository to serve the PCORnet research endeavors.”¹¹¹ that end, the primary activity of the task force during Phase I was the development of guidance around informed consent, the sustainability of biobanks, and a framework document that identified key operations concerns when preparing, handling, or storing specimens. Although these foundational documents are critical for the initial development and management of biobanks, respondents suggested that the most useful guidance for Phase II will involve a series of standards or best practices for managing biorepositories within and across networks. Given the current national focus on networks to support precision medicine, a guidance document that identifies standards for the integration of “a network of networks of biospecimen collection” is relevant to PCORnet’s continued development as a rich data network, as well to as the future of precision medicine research.

Clinical Trials Task Force. The two-pronged goals of the Clinical Trials Task force are to “provide a source of methods, standards, and quality by design principles for clinical trials using PCORnet” and to “develop the pathway for the first PCORnet interventional clinical trial until an RFA is written and a trial team is funded, then oversee the trial conduct, feeding back what is learned to the generalized knowledge base.”¹¹² To achieve these goals, the task force has focused on the development of guidance documents that will guide clinical trial design and implementation as PCORnet continues to evolve in Phase II.

Data Privacy Task Force. The purpose of the Data Privacy Task Force is to “develop a set of privacy policies to govern data sharing by PCORnet.”¹¹³ Most task force activity was, therefore, devoted to drafting policies around data privacy and security and developing guidance on the management of data issues for local networks. For example, respondents crafted guidance around the issue of data de-identification, including collection and maintenance, to support data-handling decisionmaking at the local level.

Respondents noted that some of the issues related to data privacy overlapped with issues relevant to other task forces, such as the Ethics and Regulatory Task Force and the Data Standards, Security, and Network Infrastructure Task Force, which required members to

¹¹¹ PCORnet, undated(a).

¹¹² PCORnet, undated(b).

¹¹³ PCORnet, undated(d).

collaborate more closely than with other groups in order to avoid duplication or conflict during the policy development process.

Data Standards, Security, and Network Infrastructure Task Force. The Data Standards, Security, and Network Infrastructure (DSSNI) Task Force was charged with creating the PCORI Distributed Research Network, “a functional distributed research network that facilitates multi-site patient-centered research across the CDRNs, PPRNs, and other interested contributors.”¹¹⁴ The vision for the distributed network is to “enable to conduct of observational research and clinical trials while allowing each participating organization to maintain physical and operational control over its data.”¹¹⁵

The study team interviewed several leaders of the DSSNI Task Force, all of whom identified slightly different priorities for the task force throughout the course of Phase I. One respondent suggested that the task force had a “dynamic set of priorities from the get-go,” largely as a result of PCORI’s shifting priorities as the organization developed, as well as the changing nature of the CDRN contracts. According to this respondent, “it felt like PCORI changed what it thought was most important on a weekly basis,” which made prioritization within DSSNI challenging. The other respondents felt that DSSNI’s priorities were more consistent throughout Phase I and identified specific milestones in the network development process as priorities that were largely achieved. For example, one respondent suggested that DSSNI’s priorities were threefold: (1) develop the common data model as soon as possible in order to facilitate infrastructure building, (2) onboard the CDRNs to support network implementation, and (3) test the network to identify and resolve bugs or glitches prior to national implementation. Another respondent echoed these priorities and suggested that “creating a simple network . . . getting partners to work together . . . and getting the data organized to answer a question” were the three key priorities of the DSSNI task force. All respondents suggested that the larger coordination of the task force members was a priority, since the multiplicity of voices and opinions contributing to the task force’s deliverables were not always in synch, but general project management of the group was not considered a prime output of the task force.

As Phase I drew to a close, DSSNI task force leaders felt rushed to complete data characterization. One respondent attributed the delays to PPRN timelines that pushed data characterization to the latter part of Phase I, which then provided limited time for beta testing. Without adequate time to perform testing iterations, data characterization is, therefore, behind schedule.

Ethics and Regulatory Task Force. The central goal of the Ethics and Regulatory Task Force is to “assist PCORI in addressing the ethical and regulatory issues related to research that

¹¹⁴ PCORnet, undated(e).

¹¹⁵ PCORnet, undated(e).

arise in its work.”¹¹⁶ To support this goal during Phase I, the primary activity of the task force was the development and management of the “jamboree”—a series of manuscript-writing sessions dedicated to issues in ethics and bioethics.

When the task force was first developed, the leaders of the Ethics and Regulatory Task Force identified ten issues relevant to PCORnet work that would benefit from additional consideration from a broad group of researchers and ethicists. They then acquired funding from the NIH Collaboratory to identify teams dedicated to addressing each of the ten issues and convened a meeting at which the groups could discuss their issue and develop manuscripts. At the time of the interview, all manuscripts had undergone peer review and were undergoing revisions.

According to task force leaders, the vision for the manuscripts is to “provide the foundation for understanding some of the Ethics and Regulatory issues in this space” and to develop specific guidance for targeted audiences in future iterations of the task force’s work.

Governance and Collaboration Task Force. The purpose of the Governance and Collaboration Task Force is to “assist the CDRNs/PPRNs with establishing a culture of trust and collaboration among their partners, as well as with each other, and with other external parties who participate in PCORnet activities. This will require clear governance policies and procedures that articulate the goals and purposes of the networks, establish transparent processes, and emphasize a forward thinking approach to both infrastructure development and future research activities.”¹¹⁷ Although the Coordinating Center provides broad oversight and technical assistance to CDRNs and PPRNs, the Governance and Collaboration Task Force functions as a supportive intermediary to facilitate collaboration and help interpret initial policies and procedures developed by the task force.

Health Systems Interactions and Sustainability Task Force. The goal of the Health Systems Interactions and Sustainability (HSIS) Task Force is to create “a supportive environment and sense of community across CDRNs establishing trust, common goals, and a safe forum for shared learning; promoting collaboration across Task Forces when issues overlap; and connecting CDRNs with resources both in and outside of the PCORnet Coordinating Center to help them succeed.”¹¹⁸ As a supportive task force designed to meet the needs of CDRNs and PPRNs (which asked to be included in the task force), HSIS’ first priority was to develop an inclusive membership and cultivate an environment in which CDRN and PPRN members felt welcomed. According to one respondent, this helped CDRNs to learn from one another while establishing their respective networks, which task force members considered useful.

Following the development of the task force, leaders identified network sustainability as the primary priority of the group. To best support the membership and address issues related to

¹¹⁶ PCORnet, undated(f).

¹¹⁷ PCORnet, undated(g).

¹¹⁸ PCORnet, undated(h).

sustainability, the task force performed a survey to determine what CDRNs and PPRNs hoped to accomplish within their networks and within PCORnet. The survey was then used to determine task force priorities that will be implemented going forward.

Although HSIS task force leaders felt that they had succeeded in creating a welcoming and collaborative space for task force membership, one respondent suggested that a primary challenge for the overall sustainability of PCORnet is the limited reach of participant buy-in. Specifically, the respondent noted that health care providers (physicians, nurses, etc.) will be expected to contribute to data collection activities that support PCORnet, but there has been very little outreach to these groups. Given the primary activities of HSIS, this challenge directly affects HSIS task force members and is, therefore, particularly apparent to its leadership.

Obesity/Weight Task Force. The goal of the Obesity/Weight Task Force is to “facilitate and coordinate the construction of the obesity cohort at each of the CDRNs, and to assess its feasibility, quality and interoperability with the other CDRNs and the PPRNs.”¹¹⁹ To this end, Obesity/Weight Task Force leaders identified the primary priorities of the task force to be the development of weight cohorts, creation of associated algorithms, and the oversight of obesity-related demonstration projects.

Patient and Consumer Engagement Task Force. The goal of the Patient and Consumer Engagement (PCE) Task Force is to “ensure active and effective engagement of patients and consumers in the design and implementation of all components of PCORnet.”¹²⁰ To meet this goal, the PCE Task Force prioritizes connecting with patient groups using a variety of strategies in order to meet a diversity of patient needs. In Phase I, the co-chairs of PCE developed a list of potential focus areas around patient needs and invited the PCE membership to rank which issues they felt that the task force should prioritize. As a result of this process, PCE established four different working groups designed to address these issues: data collection issues around patient and consumer concerns and protections, patient engagement policy, value propositions, and underrepresented populations.

Although leaders felt that the work groups had developed important guidance for patient and consumer engagement and had established strong relationships with some patient groups, one respondent suggested that the issue identification process could have benefited from a wider solicitation. Specifically, if the task force members had been able to contribute to the list of potential issues, the prioritized issues and eventual work groups may have developed differently and either increased the buy-in of task force members or better met patient and consumer needs.

Patient-Reported Outcomes Task Force. The purpose of the Patient-Reported Outcomes (PRO) Task Force is to develop “strategies, tools, and resources related to the measurement,

¹¹⁹ PCORnet, undated(j).

¹²⁰ PCORnet, undated(k).

collection, and analysis of patient-generated information.”¹²¹ Members of the task force evaluate and assist with the selection of PRO measures for PCORnet projects and develop best practices and assistance for CDRNs and PPRNs seeking to implement the measures in selected studies.

Rare Diseases Task Force. The goal of the Rare Diseases Task Force is to “support CDRNs and PPRNs in identifying populations, developing research priorities, designing, and implementing studies for rare diseases.”¹²² To identify primary task force priorities, leaders solicited members for issues during each of the monthly calls and tracked topics from month to month. At the time of the interview, over 60 issues had been identified, which collapsed into ten broad categories of issues. By the end of Phase I, the task force had determined that patient-friendly informed consent and a human research–compliant electronic base consent process were the primary concerns of the group. Since these issues were not specific to rare diseases, however, the task force has since shifted its orientation into more of a consultant-based role. For example, task force leaders have performed outreach to other groups to offer insight into the perspective of the rare-disease patient/research community on a variety of issues. In this way, the concerns of this community “have a presence” across the spectrum of PCORnet task forces and have an opportunity for more inclusive representation in the development of the network.

¹²¹ PCORnet, undated(l).

¹²² PCORnet, undated(m).

