



PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

# Patient-Centered Outcomes Research Institute

Cooperative Agreement Funding Announcement:  
Improving Infrastructure for Conducting Patient-Centered  
Outcomes Research

The National Patient-Centered Clinical Research Network:  
Patient-Powered Research Networks (PPRN)—Phase One

Released April 23, 2013



Action	Due Date
Release Date	Tuesday, April 23, 2013
PCORI Online System Opens	Wednesday, May 15, 2013
Pre-LOI Informational Webinar	3:00 PM (EST), Thursday, June 6, 2013
<b>Letter of Intent (LOI) Due*</b>	<b>5:00 PM (EST), Wednesday, June 19, 2013</b>
Applicants Notified of LOI Approval Status	Wednesday, July 17, 2013
<b>Application Deadline</b>	<b>5:00 PM (EST) Friday, September 27, 2013</b>
Merit Review	October–November 2013
Awards Announced	December 2013

\* LOI is required to submit an application

Notes	Due Date
Coordinating Center Announcement	Summer 2013

## I. Opportunity Snapshot

*A group of patients — all with a shared clinical condition —and their caregivers are motivated to participate in patient-centered outcomes research in order to inform better health care decisions for individuals with this condition. This group is ready and willing to engage with researchers and participate in prospective studies to better understand their condition. This group has even made strides in collecting some of these data through a protected online portal, which allows them to enter self-reported information and to access the aggregated, de-identified information of other participants. However, they are still a small group of very motivated individuals and will need to grow larger and become more representative of all patients with the condition if they are to become a legitimate site for conducting valid research studies. Moreover, they have not yet been able to obtain individual members' electronic health record information, which will be an important part of building a research database. Realizing that they do not have the resources or research experience for continued growth or to conduct investigations themselves, they begin to reach out to skilled researchers in relevant clinical areas and to organizations skilled in collecting, linking, protecting and analyzing data. This process presents some challenges. The patient group is struck by how much difficulty researchers seem to have finding patients for their studies, despite their own group's eagerness to work with them. Even with their eagerness to participate, the patient group is dismayed by the complexity of the recruitment and enrollment procedures used by most research organizations. They wonder if there are more direct and efficient ways to find interested patients, obtain their consent for participation and begin conducting research.*

### A. Purpose

Every day, patients and their caregivers are faced with crucial healthcare decisions while lacking key information that they need. The Patient-Centered Outcomes Research Institute (PCORI) was created to conduct research to provide information about the best available evidence to help patients and their providers make more informed decisions. PCORI's research is intended to give patients and their caregivers a better understanding of the prevention, treatment, and care options available and the science that supports those options. However, the nation's capacity to conduct patient-centered comparative effectiveness research (CER) quickly and efficiently remains extremely limited.

The goal of PCORI's National Patient-Centered Clinical Research Network Program is to improve the nation's capacity to conduct CER efficiently, by creating a large, highly representative electronic data infrastructure for conducting clinical outcomes research. Specifically, this program will promote a more comprehensive, complete, longitudinal data infrastructure; broader participation of patients, clinicians, health systems, and payers in the research process; and improvements in analytic methods for both observational and experimental CER. The creation of a national patient-centered clinical research network could enable the US, equipped with a learning health care system, to conduct large-scale research with enhanced timeliness, accuracy and efficiency. The core components of this network will be Clinical Data Research Networks (CDRN'S) which are healthcare delivery system-based networks that have the potential to become an ideal electronic network, without structural impediments, and Patient-Powered Research



Networks (PPRN's) which are groups of patients interested in forming a research network and in participating in research.

Through this funding announcement, PCORI seeks to support new or existing PPRN's that are comprised of patients and/or caregivers who are motivated to build an ideal network and play an active role in patient-centered comparative effectiveness research (CER). PCORI has defined the characteristics of an ideal PPRN as comprised of a patient community or group that:

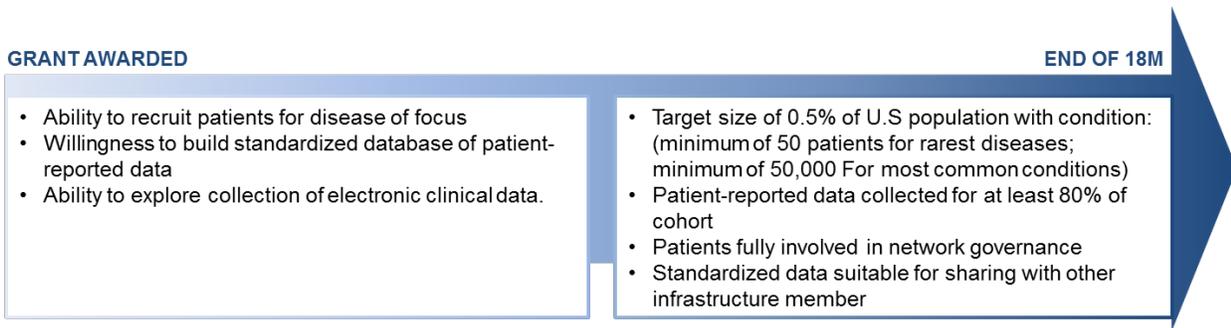
- Is comprised of patients linked by a common condition, may also include interested caregivers or clinicians, and is enthusiastic about participating in patient-centered outcomes research, including the potential to contribute research ideas, share data, adhere to protocols, and participate in observational studies and randomized clinical trials;
- Is interested in and willing to increase the quantity and quality of information collected from patients that is suitable for research from an activated patient community of at least 50,000 patients (less for patients with rare disorders);
- Has a governance structure and operating policies that ensure patient control, that can establish relationships with qualified researchers and that can generate research questions from the community's membership and accumulate relevant clinical and patient-reported outcomes data from a high proportion (at least 80%) of the membership;
- Has strategies to enhance and report the diversity and the representativeness of the patient community as it expands;
- Is interested in being actively involved in planning and conducting dissemination of research findings to patients and providers;
- Is willing to explore novel and efficient approaches for patient members to contribute their electronic clinical data to the PPRN. For example, patients could share their own data directly (obtained by providers and hospitals through the View, Download, Transmit (VDT) requirements on Meaningful Use or the Blue Button functions offered by health plans and other data holders) or ask the health care delivery sites they use to provide the data;
- Is willing to explore novel and efficient approaches for patient members to collect self-reported data, including use of remote monitoring devices, mobile apps, and self-reported observations of daily living;
- Is willing to participate in a program-wide Steering Committee that aims to share insights and approaches across the funded projects, with the aim of converging on a standards-based, inter-operable approach to building patient-powered networks and, in a subsequent funding cycle, merging them with clinical research data networks

PCORI aims to support the development of PPRNs that begin with an assembly of patients and/or caregivers, and a commitment to spread membership to all patients who wish to participate. Members of these networks must be enthusiastic about participating in patient-generated health care research, be willing to take part in studies, share data, adhere to protocols, consider participating in appropriate randomized trials and observational studies, contribute research ideas, and be actively involved in planning and conducting dissemination of research findings. In funding these PPRNs, PCORI will emphasize a collaborative approach to defining the ideal organizational structures, interfaces with researchers, standard



and condition-specific data architecture, and technology to improve patient enrollment and participation across geography, race/ethnicity, socioeconomic status, gender, and age.

Through this funding announcement, PCORI seeks to support the initiation and development of up to eighteen (18) new or existing patient research networks. During this phase PCORI will support the progression of PPRNs toward this ideal state of a reusable, scalable, and sustainable patient-centered health data research infrastructure. PCORI recognizes that existing research networks currently differ widely with respect to how closely they come to achieving these features. Thus, applicants may propose to focus efforts on enhancing different aspects of their network. The figure below articulates, on the left, the minimal requirements of an applicant organization, and on the right, the expected minimal achievement of a funded applicant at the end of Phase One.



The networks most likely to advance to Phase Two funding will be those that most thoroughly achieve all the listed features in the figure above. Of particular importance are the requirements for interoperability with other networks, involvement of patients, clinicians and health systems in governance and use of the research resource, and willingness to fully participate in a national patient-centered clinical research infrastructure. Improvements in the current health data infrastructure resulting from these completed proposals will benefit all stakeholders, including patients, caregivers, and clinicians facing healthcare decisions and researchers and, policymakers weighing the value of healthcare interventions.

### B. Funds Available

We anticipate that up to 18 PPRN awards, adding up to \$12 million in total costs will be funded under this cooperative agreement, assuming receipt of a sufficient number of high quality applications.

### C. Budget and Project Periods

Total project costs are limited to a maximum of \$1 million (total costs, including indirect costs). The project period is 18 months and may not exceed 18 months to complete work. Applicants who wish to apply for a larger award must contact PCORI by or before the due date for the letter of intent to explain why a larger award could present extraordinary opportunities to build a national patient-centered clinical research network. As part of the merit review process, budget proposals will be scrutinized and scored for the efficient use of research resources and all expenses must be clearly justified.



### D. Organizational Eligibility

We are interested in applications from patient networks that have a central goal of becoming an activated group of individuals who provide their own clinical and self-reported data for the purpose of conducting research studies that promise to improve decision-making and outcomes for patients with their condition(s). The following table provides some examples of networks that meet the eligibility criteria:

<b>Types of organizations eligible for this announcement</b>	
<b>1</b>	US-based network, group or organization of patients (either with a physical or virtual presence) of any size that has as a central goal of the establishment and growth of an activated cadre of individuals to provide their own patient-reported data for PCOR.
<b>2</b>	Networks or groups, similar to that described above, that have been developed in part through the efforts of clinicians, researchers or delivery systems to participate in comparative effectiveness research, including randomized clinical trials
<b>3</b>	Networks or groups of patients that have been convened through the efforts of internet-based or social-media based vendors, such as online communities, groups convened to use personal health records or specifically for purposes of participating in research
<b>4</b>	Existing patient-registries in which member patients are active in governance of registry activities or which aim to enlist and activate patients during this Phase One.

This is not an exhaustive list and applicants representing other types of potential PPRNs are encouraged to apply. Data vendors and researchers who can demonstrate the central involvement of patients in the governance and use of a collaborative patient network are also encouraged to submit applications. PCORI anticipates that building a PPRN will likely require work with a data vendor or possibly with a research group to provide the needed database expertise. These entities may apply as the primary institution, provided that the active engagement of the patient organization in all aspects of project leadership and governance is clearly demonstrated. Applications may be submitted by any private sector organization, including non-profit and for-profit organizations, or any public sector organization, universities, colleges, hospitals, laboratories, healthcare systems, and units of state and local governments. Only US-based organizations may apply as primary institutions. All primary applicants must be recognized by the US Internal Revenue Service. Individuals may not apply.

PCORI has a special interest in supporting research for patients with rare diseases. Registries or networks of patients with rare diseases may apply to this announcement. PCORI recognizes that the strategies necessary to build a registry or network for patients with rare diseases will vary from those for more common conditions. For example, it may be necessary to recruit patients from outside as well as inside the United States. The network sizes mentioned in this announcement may simply not be attainable for rarer conditions, and thus applicants proposing to build a network of patients with a rare disease should describe their expected sample size in terms of the proportion of all patients with the condition in the US.



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For information about the PCORI Cooperative Agreement process, please go to [application guidelines](#).

## II. Cooperative Agreement Detail

### A. Overview

PCORI is soliciting proposals for Patient-Powered Research Networks (PPRNs) in order to support the creation, development and growth of new or existing groups of motivated patients that will ultimately be prepared and willing to participate in a national patient-centered clinical research network and to work with researchers to conduct patient-centered comparative effectiveness research (CER) studies of mutual interest. PCORI may fund networks at a variety of levels of maturity, provided the proposed work under the funding announcement is a significant step forward for the network itself, a step that enhances its potential to participate in CER singly and as part of a national patient-centered clinical research network. PCORI will support collaborations of patients with researchers, and also with vendors, that can create the platform and governance to identify research opportunities that reflect patient needs, capture patient reported data in a standardized manner, and support platforms for data aggregation, analysis and transport. Patient organizations, networks or groups may independently seek out researchers and research institutions and present collaborative applications at this stage. It is expected that proposals will describe a team of individuals or entities that can collectively address the range of data collection, management, security and privacy issues required for this project. PCORI invites patient organizations with a specific disease or condition focus to work with appropriate researchers and/or with vendors who can support the creation and expansion of data resources generated from participating patients.

In an effort to realize the vision of a highly representative electronic data infrastructure for conducting clinical outcomes research, PCORI expects successful PPRN awardees to collaborate with each other, as well as with clinical data research network awardees (CDRNs) through a Steering Committee. PCORI will facilitate contacts with the CDRNs through the Steering Committee. The work in Phase One drives toward a formal collaboration of PPRNs with one or more CDRN's during a subsequent funding cycle (Phase Two).

This funding announcement targets PPRN applicants, while a companion [CDRN Announcement](#) is being released simultaneously. The work proposed in these Phase One announcements is in preparation for collaboration and linkage between CDRNs and PPRNs, which would be a central aim of subsequent (Phase Two) funding announcements. This funding constitutes the basis of the PCORI Research Infrastructure Program with the goal of creating a national patient-centered clinical research network.

### B. Background

For the first time in the US, the use of the electronic health record has spread to include the majority of ambulatory and inpatient settings, thanks in substantial part to the initiatives and policies of the



Department of Health and Human Services (HHS) through its Medicare and Medicaid EHR Incentive Programs. The policies set forth by HHS in the final rules for Meaningful Use Stage 2 represent a major step forward in advancing the secure exchange of information among clinicians and with patients to support better care across the nation. In support of Meaningful Use Stage 2, HHS adopted common standards and implementation specifications for the electronic exchange of information including common transport standards and a common dataset for the summary of care records, with an array of structured and coded data that can be formatted uniformly and sent securely to providers or patients during transitions of care or upon discharge. By 2014, clinicians will have to demonstrate, and vendors will have to support, the exchange of structured care summaries with other clinicians including across vendor boundaries. Vendors and providers will also need to support “The Blue Button Initiative” capabilities—direct patient access to and use of their own electronic health data—through the ability to view, download and transmit (VDT) their coded clinical information to a third party. EHR technology will need to enable the Nationwide Health Information Network Direct for uniform transport and Consolidated Clinical Document Architecture (CCDA) for coded content, to support the VDT requirements.

While this capability may be executed in a number of ways, some EHR technology developers are using the guidelines released by ONC for “Blue Button Plus” which satisfy Meaningful Use Stage 2 requirements and allow patients to direct an ongoing stream of their clinical data to a destination of their choice, which could include a PPRN or a specific research initiative. This could give researchers working with patients a convenient and predictable way to receive and aggregate current clinical information. From a PCOR perspective, these initiatives introduce a novel potential path to building a foundation of clinical information on an activated, engaged population of patients.

[In July 2012, PCORI convened expert stakeholders in the health data infrastructure field](#) to provide insights into opportunities for strengthening existing research infrastructure to support the conduct of high quality patient-centered CER. During this workshop, clinical data research networks (CDRNs) and patient-powered research networks (PPRNs) were discussed. CDRNs are based primarily on data derived from electronic health records and other electronic sources within a health care system, whereas PPRNs are built primarily by communities of motivated patients. Participants recognized that each type of network possesses certain advantages and agreed that an ideal infrastructure would combine the rich clinical data from large representative populations (the CDRN) with the motivation and interest in research that characterize a successful PPRN.

### **C. The Value of PPRNs**

Most clinical research to date has originated with researchers and funders, only later involving patients and their caregivers if there is a requirement for patient-reported data or active enrollment. Researchers often struggle with identifying patients, engaging them, enrolling them, collecting data and specimens from them, following these patients, sharing results with them, and including them in dissemination efforts. As a consequence, studies may fail to systematically address those research questions of most importance to patients and caregivers.



In a PPRN, control of the research process is held by the patients. The active involvement of patients in the network and in the database ensures a central role for patient participation in governing the network and its uses, identifying and selecting research questions to be studied, identifying and recruiting patients to participate in the research, and in conducting the research itself. Additional advantages include the greater potential for collection of high quality patient-reported data and for successful recruitment of members to randomized trials (when a trial is judged to be the most appropriate study).

For this Phase One of funding we encourage PPRNs to concentrate on the following activities:

- Patient recruitment to their network
- Establishment of standards-based data infrastructure and policies to support these efforts
- Characterization of the network membership in terms of demographic and clinical characteristics
- Refining the process for identifying research needs of greatest interest to patients
- Collection of clinical data from providers leveraging the View, Download, Transmit (VDT) requirements of Meaningful Use and other Blue Button efforts
- Collection of patient generated information, including patient reported outcomes information.

#### **D. Definition of Patient-Centered Outcomes Research**

PCORI has defined patient-centered outcomes research, posted the definition for public comment, and incorporated these comments into the revised [definition](#). Applications for research projects to PCORI should be aware of and aligned with this definition as they propose the data infrastructure and the involvement of patients and stakeholders in governance and use of the network resource.

#### **E. Funds Available**

We anticipate that up to 18 PPRN awards, adding up to \$12 million in total costs will be funded under this cooperative agreement, assuming receipt of a sufficient number of high quality applications.

#### **F. Budget and Project Periods**

Total project costs are limited to a maximum of \$1 million (total costs, including indirect costs). The project period is 18 months and may not exceed 18 months to complete work. Applicants who wish to apply for a larger award must contact PCORI by or before the due date for the letter of intent to explain why a larger award could present extraordinary opportunities to build a national research infrastructure. All budget proposals will be scrutinized and scored for the efficient use of research resources as part of the merit review process and all expenses must be clearly justified.

### **III. The Technical Proposal**

The technical proposal should be carefully organized according to the sections described below. In each section, except for Section 1, the applicant should describe what the network proposes to do during the 18-month period to make progress toward the ideal network. Goals, milestones and deliverables should be described. PCORI recognizes that not all applicant networks will be able to propose activities in every area.



If your organization does not envision meaningful opportunities to make progress in one of these areas, state this clearly.

## A. The Technical Proposal and Review Criteria (RC)

### 1. Description of the patient or patient/caregiver network

Describe your network or group's history, the disease(s) or condition(s) that defines membership, the network's mission, current size (number of members), recent rate of growth in membership, and current data holdings (if applicable). If available, provide information (one page) regarding the demographic and clinical characteristics of the current members. Of particular importance are data on rates at which network participants are retained and remain active in network activities year over year. Mention supporting organizations and briefly cite key relationships (e.g., with researchers, data vendors, patient advocacy organizations, health care systems, specialty societies).

Describe the current standing of your network with major patient organizations or other organizations, and current or past experience of the network in conducting research.

RC1.

- a) The application clearly describes the history and current status of the network relative to the characteristics requested.
- b) The clinical and demographic characteristics are clearly presented to the extent available.
- c) The application summarizes any clinical research projects that have been conducted within the network.

### 2. Building a clinical database of patient-contributed data

Describe the network's plans to build a clinical database that collects standardized member-reported data on patients with a specific condition for the purpose of conducting research. This information should include basic demographic information, robust contact information, clinical information pertinent to the condition, and patient-reported data on disease characteristics, severity and relevant outcomes. Demonstration of the ability to collect this patient-generated information from a large proportion (i.e., a minimum of 80% of members) is an essential requirement during Phase One, and applicants should plan and budget to accomplish this. The applicant should:

- Describe the approach to building the standardized clinical research database, including considerations related to the software platform, general and disease-specific content of the database, and proposed data standards.
- Describe the approach to efficient collection of data, including the potential use of web portals, mobile technologies and other devices that enable convenient methods of real-time data collection. For the collection of outcomes data, describe the use of standard, well-validated outcomes instruments whenever possible. If that is not possible, describe the rationale for selecting an alternative instrument.

- Describe the approaches that would leverage patients' ability to access their own electronic health record and administrative data through nationally identified standards (e.g., the VDT requirements of Meaningful Use and health plan and other data holders' Blue Button efforts.)
- Describe how the PPRN will participate in national standards-development activities with other PPRN and CDRN awardees through the Steering Committee to build toward a scalable, inter-operable national patient-centered clinical research network.

RC2.

- a) The application clearly describes the current capacity of the network to collect data on patient-generated and patient-reported outcomes and efficiently contact patients for recruitment into prospective studies, including clinical trials. Examples of past or current accomplishments in terms of data collection, standardization or analyses should be given, if applicable.
- b) The application describes plans to enhance this capacity, including through the use of surveys, portals and mobile health devices, patient engagement, development of policies and practices that facilitate health provider and patient contact for research purposes.
- c) The application describes awareness of the need to identify a consistent set of data standards across the funded networks and willingness to participate in these activities.
- d) The application demonstrates awareness of the potential for accessing clinical care delivery data through use of data sharing requirements in Meaningful Use and Blue Button initiatives.

### **3. Plans for increasing the size, diversity and representativeness of the network**

Each PPRN is expected to work during the 18-month period not only to expand its membership, but to increase the diversity and representativeness of its network. It is expected that for many conditions, initial networks of volunteers will be highly motivated to participate in research but these volunteers may not be a highly representative sample of all patients with the condition. Attention to broadening participation in the network is a key priority during Phase One. The applicant should:

- Describe the expected size of the PPRN in terms of numbers of members by the end of the 18-month award period.
- Describe the network's plan to reach out to and enroll other patients, especially those from historically under-represented groups defined in terms of race/ethnicity, socioeconomic status, geographic location, health literacy, clinical severity and other characteristics (as appropriate).
- Describe specific steps planned to work with patient advocacy organizations or healthcare providers to increase enrollment. Discuss particular barriers to enrollment, innovative solutions your network will employ to reach additional members and the expected network size at the end of the period.
- Describe how you will assess and report on the representativeness of the assembled network of patients near the end of the 18-month period, and steps you will take to retain participation over time by participants once they have enrolled.

RC3.

- a) The application demonstrates that the network has or will build the capacity to rigorously identify, engage and retain members of these cohorts and that the cohorts will be of sufficient size and composition to support meaningful studies. The application proposes the 18-month objective of building a network size that is proportional to the numbers of patients with the condition in the US population. Except for conditions at the extremes of prevalence (the rarest and the most common), a useful target would be to capture at least 0.5% of the patients with the condition in the US. For the rarest conditions, the minimum requirement will be 50 patients; for the most common conditions (those with a prevalence of 3% or more in the entire population) a minimum requirement to achieve at the end of Phase I will be 50,000 patients.
- b) The plan presented for expanding the network's size and recruiting a broader, more representative population is reasonable and likely to succeed.
- c) The application clearly describes an understanding of the importance of enhancing and documenting the representativeness of the patient population, in terms of demographic and clinical characteristics.

#### **4. Plans for involving patient participants in network governance**

PCORI expects that each PPRN will be governed substantially by its participating members, and that group leadership will be representative, able to receive and act upon broad input of participating individuals, have policies in place to negotiate divergent opinions, and that this governance structure will be well established by the end of the 18-month period. This expectation holds regardless of whether the primary award recipient is the network itself or an associated research institution or vendor organization. PCORI will look to the PPRNs in developing these policies and approaches and explore ways to transfer best practices to building patient activation and participation within the larger CDRNs. The applicant should:

- Describe the current governance structure within the group or network, including the level of patient representation.
- Describe existing policies regarding how to engage network members more fully in governance decisions. Key decisions include those related to the mission of the network, the relationships it establishes with researchers and others, the studies and data sharing arrangements it chooses to participate in, the ways in which members may use the network to raise and answer research questions, and the strategies to be used for disseminating and promoting findings. Speak to the organizational structure for the network, its methods for communication among members, and policies for decisions related to study participation and data sharing.
- Describe plans for facilitating communications among network participants — e.g., for purposes of sharing experiences, generating clinical or research questions, and for discussing potential research opportunities presented to the network.

RC4.



- a) The application describes how the network plans to engage patients in the governance of the PPRN, including written policies for decision-making about network participation in proposed studies.
- b) The application has a clear plan for involving network members in generating research questions and in participating in research studies.
- c) The proposed infrastructure and strategies for communication among network members and for inviting participation from patients in research studies are substantial, feasible, and well described.

**5. Willingness to participate in a national patient-centered clinical research network for the conduct of CER in collaboration with researchers affiliated with CDRN awardees, as well as with researchers from non-affiliated organizations.**

PCORI's intention is to build a national resource for conducting CER. Though PCORI awardees will provide the foundation for this resource, this work is meant to enable future research to be efficiently conducted through the collaboration of CDRNs and PPRNs both within PCORI's network and outside of it. Therefore, to be eligible for Phase Two funding, PPRNs must demonstrate a willingness and capacity to collaborate with CDRNs and to participate in studies led by qualified researchers from outside the network, and to actively participate in research studies that are of interest to its members. These formal research collaborations would not be expected until the PPRN achieves a level of maturity and size that can support research, which may not be until very late in the Phase One funding period. However, PPRNs are expected to work closely with CDRNs throughout Phase One to assist them in incorporating the patient voice into their networks and into the national clinical research network. The Steering Committee and the Coordinating Center will play important roles in enabling these collaborations. Applicants should plan for an important time commitment to work with the Steering Committee and the Coordinating Center throughout the process, including weekly telephone calls and quarterly face to face meetings during the 18 month period. Budgets should include travel for these meetings.

The expectation to collaborate does not supersede or overrule the judgment of the PPRN's patient governance structure in making choices about study participation. The applicant should:

- Describe the general willingness of the PPRN to collaborate with other components of the network, such as one or more CDRNs in research activities, including activities involving researchers from outside the network, once the PPRN's size and clinical database can support research activities
- Demonstrate a willingness and insight on the part of the PPRN as to how PPRNs can support CDRNs in enhancing patient engagement activities within large CDRNs, using processes and policies developed first within PPRNs.

**RC5.**

- a) The application conveys an understanding of PCORI's intentions to create a national patient-centered clinical research network and the individual network's willingness and interest in participating in this national resource.
- b) The application presents a coherent plan to work toward data standards and polices that

would support efficient collaborations with CDRNs in conducting CER, including observational and randomized studies.

- c) The application clearly describes preparation of policies, procedures and processes to create standardized data and make it available for analysis through central pooling and/or federated access.

## 6. Creation of a standardized network model

PCORI is interested in funding networks that can serve as models for patients with other conditions. A key requirement is the collection and storage of data using data definitions, formats and data architecture that allow for efficient and accurate data sharing. We expect that the awardees will work through the Steering Committee toward identifying a data standardization approach that will support the efficient storage, access and expansion of individual network databases, the exchange of standardized information between networks, and the aggregation of data, directly or virtually, for purposes of analyses. The applicant should:

- Describe data standardization and quality control practices that are already in place within your network's data systems, if applicable.
- Describe the data standardization experience of the network to date and efforts that are proposed to further increase the degree and scope of standardization, including use of standardized registry architectures, data elements and definitions.
- Describe your familiarity with any emerging national and international standards in diagnosis, procedure, and pharmaceutical coding, or for vocabulary or interoperability, including patient-reported outcomes, as appropriate for the disease that is the focus within your network.

RC6.

- a) The application demonstrates an ability to describe the data structure of the existing or planned network database,
- b) The application describes an awareness of the key technical issues related to data standardization, data sharing, data quality and harmonization and existing efforts at standardization appropriate to the disease being studied.

## 7. Ability to share data with external organizations, and to implement data-sharing agreements

Awardees are expected to develop policies and capacity to allow for efficient sharing of data with other PPRN and CDRN awardees and with external collaborators by the end of the 18-month period. This requires governance policies for approving and overseeing the sharing of data, obtaining of informed consent from network members. Capabilities for de-identifying and re-identifying data and for secure transmission must be developed. It is expected that these capabilities and policies would be developed in collaboration with other awardees through the Steering Committee. Describe any experience your network has had in these areas, any concerns, and the steps you plan to take toward this goal. The applicant should:

- Describe your approach to protecting human subjects in the conduct of research, including arrangements you have in place for data security and privacy, de-identification and re-identification plans,
- Describe the network’s approach or planned approach for obtaining informed consent for the storage, use within the network, sharing of data outside the network, and future re-use of self-reported and clinical information. Include milestones and deliverables related to this work.
- Describe the extent to which this process has been reviewed and approved by an institutional review board, and describe and budget for the work that remains to be accomplished.
- Also describe how you will develop policies that reinforce transparency about patient involvement in research including patient access to research results, policies around re-use of data and how researchers will be expected to engage patients in and communicate with them about their research. This includes description of reasonable efforts made to ensure that patients are aware of the variety of uses of their data and the outcomes of these uses.

RC7.

- a) The application describes the network’s approach to sharing of data.
- b) The application describes plans to increase data-sharing capacity in a secure, standardized way; including the capacity to de-identify and re-identify data.
- c) The application describes the network’s plans for developing the policies needed to ensure data security, protection of human subjects, and obtaining informed consent related to conducting research within network and to data-sharing outside of the PPRN, including milestones and deliverables.
- d) The application describes the network’s current and planned approach to obtaining informed consent from members and any experience to date with involvement of an institutional review board and human subjects oversight.
- e) The application describes plans to communicate with patients about the research and research results.

**8. Sufficient administrative and financial accounting structures to be able to receive, manage and account for contract funds and experienced team members.**

PCORI envisions that a mature national patient-centered clinical research network will be a lean, efficient and sustainable endeavor. This requires efficiencies within each participating network (i.e., each PPRN). All prime contractors, whether a patient organization or a research institution or vendor organization, should demonstrate the administrative and financial capacity to carry out all PPRN deliverables. Applicants should also describe their team and emphasize relevant experience to successfully carry out the project. The applicant should:

- Describe the capacity and track record of the primary applicant organization in accepting and successfully managing research contracts.

- Describe how the budget requested reflects the efficiency of the network and how the network will maintain this efficiency after the growth and expansion accomplished under PCORI funding.
- Describe the project team and highlight relevant experience in this type of endeavor. The team should include a range of individuals with relevant skills and experience to address the variety of issues at hand.

RC8.

- a) The application describes clearly the financial and accounting structures of the entity that will be the prime contractor.
- b) The proposed budget is justified for each 6-month period of the project, in terms of resources and activities associated with the work tasks described.
- c) The application provides a clear and well organized explanation of the human, technical and organizational resources that will have to be put in place and the work tasks that will have to be completed in order to achieve the goals, milestones and deliverables proposed, during the 18 months of the project.
- d) The application describes the team members and their relevant experience and skills in this area.
- e) The application describes attention to future efficiency, value and sustainability in the operation of the network and its participation in a national patient-centered clinical research network.

#### **9. Capacity to obtain standardized Electronic Health Record (EHR) data on consenting network members**

A key goal of this effort is to explore strategies for the efficient collection and merging of the EHR data of network members, obtained from their clinical providers (hospitals, and ambulatory settings) with the network's patient-generated data. Health plan data can also contribute to building each patient's longitudinal record. Numerous challenges exist. Network members are likely to be covered by a range of health plans, and to receive care from multiple provider systems. Some providers may still not have EHRs. The applicant should:

- Describe their plans and associated budgets for supporting patients in obtaining their clinical data from providers and hospitals through standards-based approaches (i.e., meaningful use view, download and transmit requirements, or Blue Button functions offered by health plans and other data holders).
- If other strategies such as working with specific health plans, healthcare delivery systems, or data vendors experienced in this area are used, describe how you will develop standards-based approaches to obtain high quality clinical record information for patients that have authorized this access.
- Specific goals of the effort should be listed as well as the challenges anticipated.

RC9.



- a) The applicant demonstrates familiarity with the potential options for obtaining clinical data from EHRs and other electronic sources, including the opportunities posed by Meaningful Use VDT and Blue Button functions
- b) The application clearly describes current policies and practices to ensure data security, in collecting, storing and using clinical data, including those required to adhere to federal, state and health system requirements.
- c) The application demonstrates the willingness and interest of the network in pursuing various options for obtaining clinical data on network members.

#### **10. Capacity to store genetic data or biomarkers, as well as apply this information for purposes of PCOR**

The ability to collect and store biospecimens is not a requirement of applicant organizations for this Phase One announcement. However, PCORI's longer-term vision for a national patient-centered clinical research data network includes the capacity to efficiently collect and store biospecimen data. Applicants should describe any prior experience in this area, their level of interest and that of their network's membership, and any anticipated barriers to collection, storage and sharing of biospecimens (e.g., saliva, blood, serum, or tissue) for research purposes, including collection of patient informed consent for future uses of the specimens. Other considerations being equal, networks with the capacity or interest in pursuing collection of biological specimens will be scored favorably.

RC10.

- a) If applicable, the application clearly describes the current and end-state capacity of the network to acquire, and store biospecimens and collect genetic data and/or biomarkers.

#### **B. Review and Selection Process**

PCORI conducts rigorous merit reviews of all applications for all PCORI Funding Announcements (PFAs). In order to promote quality scientific research, while ensuring patient-centeredness, the PCORI merit review is distinguished by the full participation of scientists, patients and their caregivers, and other stakeholders in the process. The review process includes online review, in-person merit review and post-panel assessment. You can learn more about the PCORI standard review process [here](#).

PCORI's merit review panels are composed of scientific reviewers, patients, and other stakeholders. At least 30% of review panel members are non-scientists. Moreover, not all scientists will be specialized in the topic of your research. Therefore, applicants are strongly advised to write their applications as clearly as possible without losing scientific meaning. Although applications will not be directly scored on the clarity of writing, clearly written proposals will have an advantage. Particular attention should be paid to the clarity of the lay abstract and to discussions of patient-centeredness and the engagement of patients and other stakeholders on the research team.



A summary of the general review process is as follows:

1. Full proposals receive an internal review by PCORI staff
2. Online External Review: Reviewers provide qualitative critiques and quantitative scores for the applications. Each application will be reviewed by two external scientists, one patient and one stakeholder
3. In-person merit review: Final review and discussion by external reviewers
4. PCORI Scientific Staff makes preliminary funding recommendation to PCORI selection committee\*
5. PCORI selection committee make final funding recommendation to PCORI Board of Governors (BoG)
6. PCORI BoG make final decision
7. PCORI staff conducts a business review to evaluate and possibly negotiate aspects of the budget

\* PCORI selection committee includes PCORI Board of Governors members, PCORI Executive Leader and PCORI Scientific Personnel.

You should carefully read and thoroughly understand the PCORI review criteria as listed in the description of each technical requirement above. Participants in the merit review process will be asked to use these criteria in evaluating and scoring applications.

#### **IV. Application and Submission Guidelines**

Please refer to the Application and Submission Guidelines document [here](#) for detailed instructions of how to submit your LOI and full application via PCORI Online System.

##### **A. Letters of Intent (LOI):**

PCORI recognizes that the range of applicants to this announcement may be broad and diverse and that not all potential applicants may be a good fit at this point. To reduce excessive work on the part of applicants and to reduce the time and resource requirements for technical review, prospective applicants for Phase One must submit a LOI online via PCORI Online System. *Applicants are also encouraged to contact PCORI staff before and after submission of the LOI to ensure production of optimally responsive applications.* LOIs will be reviewed based on the fit of the applicant network with the goals of the PFA, and feasibility to complete work within budget and project period proposed. Applicants are encouraged to submit the LOI as early as feasible, rather than waiting for the submission deadline for LOIs. We emphasize again that new networks are encouraged to apply.

##### **B. Funding and Project Period Limits**

A range of funding is available, from \$250,000 to \$500,000 over 18 months for smaller and newer groups in the early stages of development. Smaller and more recently organized patient entities are encouraged to



apply. For more established patient organizations or networks (with potential to create networks of 25,000 or more members) up to \$1 million per award will be available. Proposed networks of patients with rare diseases (with a prevalence of less than one in 1,500 persons in the US, or less than approximately 200,000 total patients in the US) are not bound by this size requirement. The budget should also be consistent with the targeted number of members by the end of the 18-month period. Organizations or networks that could provide particularly attractive opportunities for the national research network, but would require more than \$1 million to do so, should submit their request for permission to submit the larger request at the LOI due date but they are encouraged to submit earlier as this request might require longer to review. As one example, an organization that proposed to coordinate the participation of two or more patient networks would be of interest. PCORI does not anticipate granting such requests unless the proposed activity represents an extraordinary opportunity to obtain novel information that could generalize readily to other PPRNs.

Efficient use of research resources is a criterion that will be considered by merit reviewers and will also be reviewed by PCORI staff. The total amount awarded and the number of awards will depend on the quality, duration, and costs of the applications received.

*PCORI also reserves the right to discontinue funding for awardees that fail to meet the mutually agree upon milestones at any time during the award. In addition, a go/no-go decision will occur after six months of the award.* Proposed milestones should be presented in the application, but final milestones will be negotiated in the post-award period prior to the beginning of the funding period. Details of this policy will be outlined in the contract of these awards.

## About PCORI

The Patient-Centered Outcomes Research Institute (PCORI) is an independent organization created to help people make informed health care decisions and improve health care delivery. PCORI will commission research that is guided by patients, caregivers, and the broader health care community and will produce high integrity, evidence-based information.

PCORI is committed to transparency and a rigorous stakeholder-driven process that emphasizes patient engagement. PCORI will use a variety of forums and public comment periods to obtain public input throughout its work.

**Our Mission:** PCORI helps people make informed health care decisions and improves health care delivery and outcomes by producing and promoting high integrity, evidence-based information that comes from research guided by patients, caregivers, and the broader health care community.

**Our History:** PCORI was created by the Patient Protection and Affordable Care Act of 2010 as a non-profit, nongovernmental organization. PCORI's purpose, as defined by the law, is to help patients, clinicians, purchasers, and policy makers make better informed health decisions by "advancing the quality and relevance of evidence about how to prevent, diagnose, treat, monitor, and manage diseases, disorders, and other health conditions."

The statutory language defining PCORI is broad and authorizes research that will support a strong patient-centered orientation, inform better choices among alternative treatment and prevention strategies, and direct attention to individual and system differences that may influence strategies and outcomes. PCORI was designed to produce knowledge through the analysis and synthesis of existing research or the support of new research.