The National Patient-Centered Clinical Research Network:
Building a National Data Infrastructure to Advance Patient-Centered Comparative Effectiveness Research (CER)

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PCORI’s Mission and Vision

- The Patient-Centered Outcomes Research Institute (PCORI) is an independent, non-profit health research organization authorized by the Patient Protection and Affordable Care Act of 2010.

- PCORI funds patient-centered research to assist patients, caregivers, and other stakeholders in making informed health decisions.

**Mission**

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

**Vision**

Patients and the public have the information they need to make decisions that reflect their desired health outcomes.
PCORI’s Three Strategic Goals

- Substantially increase the quantity, quality, and timeliness of useful, trust-worthy evidence available to support health decisions.
- Influence clinical and health care research funded by others to be more patient-centered.
- Speed the implementation and use of patient-centered outcomes research evidence.
PCORI’s unique contribution to this field of research is its dedicated focus on research questions, study populations, and outcomes that are relevant to patients.

Inclusion of outcomes that matter to patients is a necessary component of PCORI-funded research.

PCORI involves patients and the entire healthcare community in every step of the research process — from selecting and prioritizing research questions to study, to the actual conduct of research to sharing the research findings with key stakeholder communities to support implementation.
PCORI’S National Priorities for Research

- Assessment of Prevention, Diagnosis, and Treatment Options
- Improving Healthcare Systems
- Communication & Dissemination Research
- Addressing Disparities
- Accelerating PCOR and Methodological Research
National Patient-Centered Clinical Research Network
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, national patient-centered clinical research network** for conducting clinical outcomes research.

The **vision** is to support a learning US healthcare system, which would allow for **large-scale research** to be conducted with **enhanced accuracy and efficiency**.
National Patient-Centered Clinical Research Network

The core components of this network will be:

- Clinical Data Research Networks (CDRNs), which are system-based networks that have the potential to become an ideal electronic network.
- Patient-Powered Research Networks (PPRNs), which are groups of patients interested in forming a research network and in participating in research.
- A Coordinating Center which will provide technical and logistical assistance under the direction of the Steering Committee and PCORI Staff.

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.
- Improvements in analytic methods for both observational and experimental CER.
National Patient-Centered Clinical Research Network: Our Vision

Steering Committee
- Awardees
- PCORI
- AHRQ, NIH, FDA, ONC, CMS

Scientific Advisory Board

Special Expert Group

Coordinating Center Staff
National Patient-Centered Clinical Research Network Coordinating Center

- Led by Harvard Pilgrim Healthcare and Duke Clinical Research Institute

- AcademyHealth, AHIP, Brookings Institution, Center for Democracy and Technology, Center for Medical Technology Policy, Group Health Research Institute, Johns Hopkins University, RAND Corporation.

- Supports CDRNs and PPRNs
  - Project Management
  - Technical Assistance
  - Meeting support for cross-awardee activities
  - Program evaluation
National Patient-Centered Clinical Research Network: Supporting Resources

Steering Committee (SC)
- Makes decisions, generates policies and develops best practices and methods
- Comprised of representatives from PCORI staff, the CC, federal agencies, awardees

Scientific Advisory Board (SAB)
- Provides insight and guidance on the development of the national research infrastructure
Special Expert Group (SEG)

- Provides insight about recruitment and retention, data richness, completeness and quality, inter-operability across systems and networks, and data standardization
National Patient-Centered Clinical Research Network: Key Areas of Focus

- Data Standards and Interoperability
- Governance / Collaboration
- Health System Leadership Involvement / Sustainability
- Patient Engagement
- Patient Reported Outcomes
- Ethical Oversight
- Privacy and Security
- Biospecimens and biorepositories
- Obesity workgroup
- Rare disease workgroup
Ideal PCORI CDRN Characteristics
Ideal CDRN Characteristics: Systems

- Involvement of multiple (two or more) health systems, working toward data standardization and interoperability within the network and across networks to allow for efficient, valid sharing of individual or aggregate data for purposes of data analysis.

- Involvement of the healthcare system leadership in governance and use of the network to enhance network efficiency, utility, and identification of models for sustainability of the network.

- Capacity and willingness to support large-scale randomized trials, as well as observational comparative effectiveness studies, with substantive patient and clinician involvement in the governance and use of the network.
Ideal CDRN Characteristics: Patients

- **Coverage of a large, diverse, defined population** not selected for a particular disease, condition, or procedure; **ability to capture complete clinical information on this population** over time, including longitudinal information on clinical care, changes in clinical characteristics and conditions, and the occurrence of clinical care or outcomes, within or outside the system.

- The **ability to efficiently contact patients** for the purposes of recruitment; collecting patient-reported information; and maintaining **consistently high levels of participation in research studies**

- Demonstrated **ability to engage substantial patient populations** with selected conditions, both within and outside their systems, for purposes of generating research questions, participating in network governance, or in appropriate research studies.
Willingness to **serve as a part of a national data infrastructure resource** for the conduct of CER by researchers within or outside the network.

**Capacity to embed research activity within functioning healthcare systems** without disrupting the business of providing health care;

Alignment of human subjects oversight, IRB review and approval, and informed consent procedures with the level of risk in proposed studies

**Clear, proven policies and track record for maintaining data security, patient privacy, and confidentiality;**

Ability to collect, store, retrieve, process, or ship biological specimens for research purposes, with appropriate consent, for use by qualified researchers.

Ability to **streamline subcontracting processes** for research involving multiple sites.
Clinical Data Research Networks (CDRN)

$56 million to support up to 8 new or existing CDRNs for 18 months

**COOPERATIVE AGREEMENT AWARD 18 MONTHS LATER**

- At least two health care systems engaged
- Willingness and capacity to work toward data standardization with other awardees
- Willingness to participate in collaborative studies with data sharing as part of a national research infrastructure

- > 1,000,000 patients enrolled
- Data standardized within network and with other awardee networks
- Patients, system, and clinicians engaged in governance & use
- Capable of implementing clinical trials
Ideal PCORI PPRN Characteristics, Activities to Focus On
Ideal PPRN Characteristics: Patients

- Is comprised of patients linked by a common condition, may also include interested caregivers or clinicians, and is enthusiastic about participating in research.

- Is interested in and willing to increase the amount of information collected (suitable for research) from an activated patient community of at least 50,000 patients (less for patients with rare disorders).

- Is willing to explore new approaches for patient members to contribute their electronic clinical data to the PPRN (e.g. use of Blue Button technology).

- Is willing to explore new approaches for patient members to collect self-reported data.
Ideal PPRN Characteristics: Systems

- Is willing to participate in a program-wide Steering Committee to help resolve network-wide challenges
- Is willing to collaborate with other awardees, with the aim of:
  - converging on a standards-based, inter-operable approach to building patient-powered networks
  - merging in a subsequent funding cycle, with clinical research data networks
Ideal PPRN Characteristics: Administrative

- Has a governance structure and operating policies that ensure patient control, and can establish relationships with qualified researchers.
- Can accumulate relevant clinical and patient-reported outcomes data from a high proportion (at least 80%) of the membership.
- Is interested in being actively involved in planning and conducting dissemination of research findings to patients and providers.
- Has strategies to enhance and report the diversity and the representativeness of the patient community as it expands.
PPRNs Should Concentrate on the Following Activities During the 18 months Initiative

- **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.
Patient-Powered Research Networks

$12 million is available to support up to 18 new or existing PPRNs for 18 months.

**COOPERATIVE AGREEMENT AWARD → 18 MONTHS LATER**

- Patients with a single condition, interested in research participation.
- Ability to increase size and diversity of patient membership
- Willingness to build standardized database of patient-reported data
- Willingness to explore collection of electronic clinical data.

- Target size of 0.5% of U.S population with condition: (>50 patients for rarest diseases; 10,000 for most common)
- Patient-reported data collected for at least 80% of cohort
- Patients involved in governance
- Standardized data suitable for sharing with other infrastructure members
CDRN and PPRN Applicants
28 CDRN Applications

- Non-profit integrated health systems
- Major commercial health plans
- Military health plans
- Networks of academic medical centers, hospitals and physician practices
- Networks of low income clinics
- Networks leveraging AHRQ investments
- NIH investments
- Network based on HIEs
- Geographical representation
- Underserved populations
- Range from 1M covered lives to 12M
Variety of **stakeholders** in participating organizations and in leadership team: patients, advocacy groups, physician organizations, academic centers, PBRNs etc.

Strong understanding of **patient engagement**

Significant range of **conditions and diseases**

Variety in **populations** represented (including pediatrics, underserved populations etc.)

Close to 50% **rare diseases**

Significant range in the **maturity** of the group (some with little data and some with significant data collected from registries or databases),

**Biospecimens**
Advancing to Phase 2

Networks that meet the detailed requirements in the funding announcement, including

- **interoperability** with other networks,
- involvement of patients, clinicians and health systems in **governance** and use of the resource,
- willingness to **fully participate** in a national patient-centered research network.
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