



Building a National Data Infrastructure to Advance Patient-Centered Comparative Clinical Effectiveness Research

Joe V. Selby, MD, MPH, Executive Director

Patient-Centered Outcomes Research Institute

About PCORI

An independent, non-profit research organization authorized by Congress as part of the 2010 Patient Protection and Affordable Care Act (PPACA)



PCORI funds comparative clinical effectiveness research that provides patients and those who care for them the information they need to make better informed health care decisions.

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

Accelerating PCOR and Methodological Research

- Improving study designs and analytic methods of PCOR
- Building and improving clinical data networks
- Methods for training researchers, patients to participate in PCOR
- Establishing methodology for the study of rare diseases

Distinct (Complementary) Approaches Emerge

**PCORI National Workshop to
Advance Use of Electronic Data
Palo Alto, CA
July 2-3, 2012**

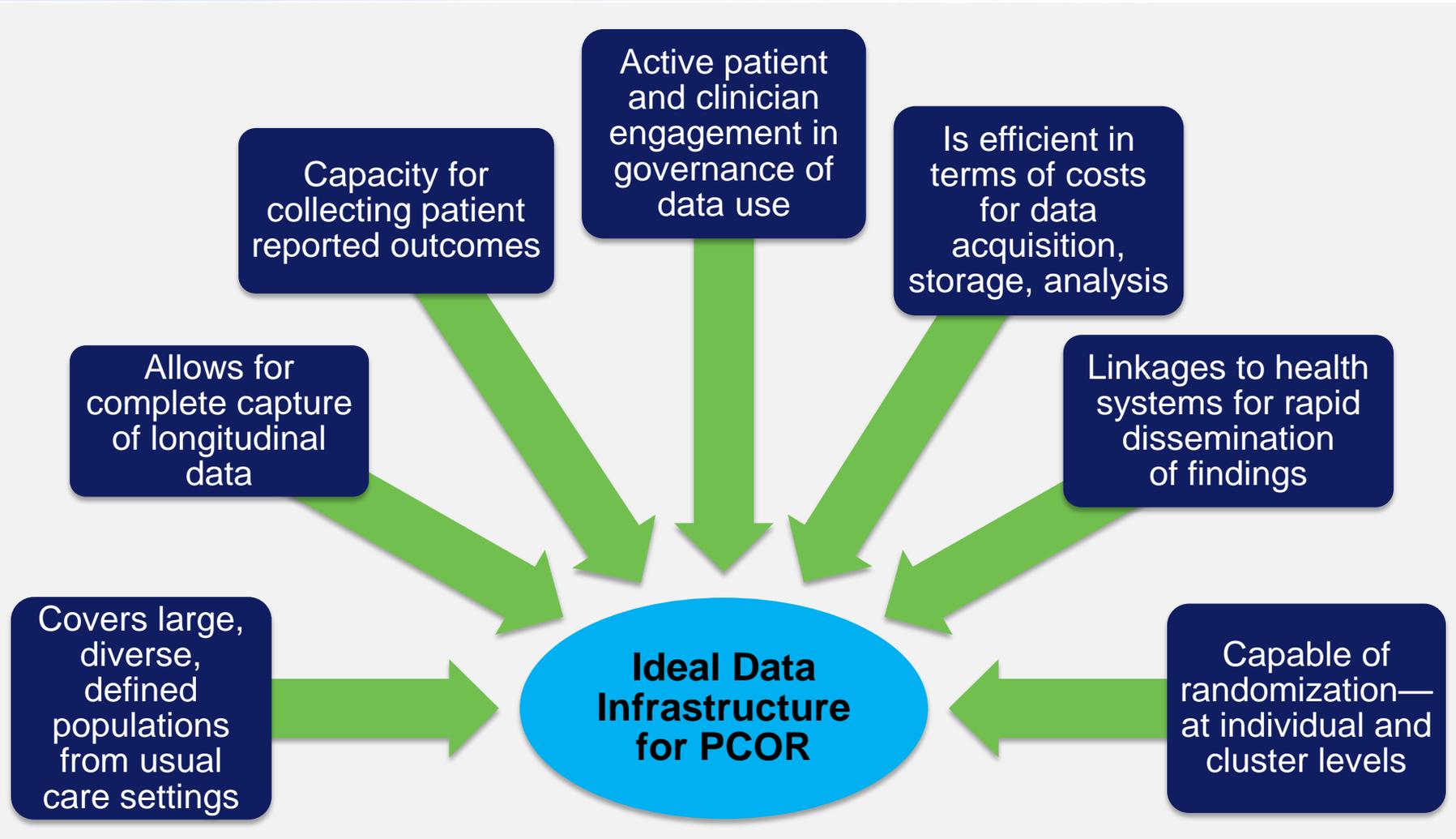


**Clinical Data
Networks**

**Patient-
Powered
Research
Networks**



Desirable Characteristics of a Data Infrastructure for Patient-Centered Outcomes Research



National Patient-Centered Clinical Research Network: Our Vision

Builds on:

- Recent widespread implementation of electronic health records in ambulatory and hospital settings
- Previous and ongoing efforts to build research networks
- Growing interest of patient organizations to participate in and govern clinical research activities
- Growing awareness of health care delivery systems and clinicians of the need for good research evidence drawn from clinical practice

Previously Funded Research Networks



ONC

- Meaningful Use
- Nationwide Health Information Network
- Federal Health Architecture
- EHR Certification Standards & Interoperability Framework



FDA

- Mini-Sentinel
- OMOP



AHRQ

- DRNs
- PBRNs
- Registries
- SPAN
- PROSPECT
- EDM Forum



NIH

- SEER Registries
- CTSA
- Collaboratory
- CRN, CVRN
- eMERGE Network
- PROMIS/ NIH - Snomed-CT, LOINC



VA

- VistA
- iEHR (2017)



IOM

2011 Report: *Digital Infrastructure for the Learning Health System: The Foundation for Continuous Improvement in Health and Health Care*

Payers



Specialty Societies



Industry



Innovators and Entrepreneurs



National Patient-Centered Clinical Research Network: Our Vision

Characteristics:

- A “network of networks”
- Two types of component networks: systems-generated and patient driven networks
- Active involvement of health care systems, of clinicians and of patients
- A commitment to establishing inter-operability and data sharing across networks and ready collaboration with the larger research community

National Patient-Centered Clinical Research Network: Our Vision

Capabilities:

- Rich clinical data from electronic health records and other data sources stored in standardized, interoperable formats.
- Capacity to conduct both observational studies and randomized trials embedded in clinical settings
- Rigorous practices for data security and confidentiality
- Appropriate IRB and human subjects oversight
- Utility for comparative clinical effectiveness, safety studies, surveillance, etiologic research, and potentially for pre-approval trials

Funding Opportunity

- Two funding announcements for up to \$68 million to support development of a National Patient-Centered Clinical Research Network.
 - Clinical Data Research Networks (CDRN)
 - Patient-Powered Research Networks (PPRN)
- Key Dates:
 - April 23, Funding Announcement Release Date
 - June 19, Letters of Intent (LOI) Due
 - September 27, Application Deadline
 - December, Awards Announced

Clinical Data Research Networks (CDRN)

- \$56 million to support up to 8 new or existing CDRNs for 18 months to expand capacity for conducting efficient CER studies, both randomized and observational, across multiple CDRNs using data from clinical practice in large, defined populations

COOPERATIVE AGREEMENT AWARDED

END OF 18 MONTHS

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

- $\geq 1,000,000$ patients enrolled
- Data standardized within network and with other awardee networks
- Patients, system, and clinicians engaged in network governance & use
- At least three patient cohorts identified, characterized, and surveyed
- Capable of implementing clinical trials

Patient-Powered Research Networks (PPRN)

- \$12 million to support up to 18 new or existing PPRNs, their progression toward larger networks, and their collaboration with CDRNs.

COOPERATIVE AGREEMENT AWARDED

END OF 18 MONTHS

- Patients with a single condition, interested in research participation.
- Ability to increase size and diversity of patient membership with a disease of focus
- 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: (minimum of 50 patients for rarest diseases; 10,000 for most common)
- Patient-reported data collected for at least 80% of cohort
- Patients fully involved in network governance
- Standardized data suitable for sharing with other infrastructure members

National Patient-Centered Clinical Research Network: Our Vision

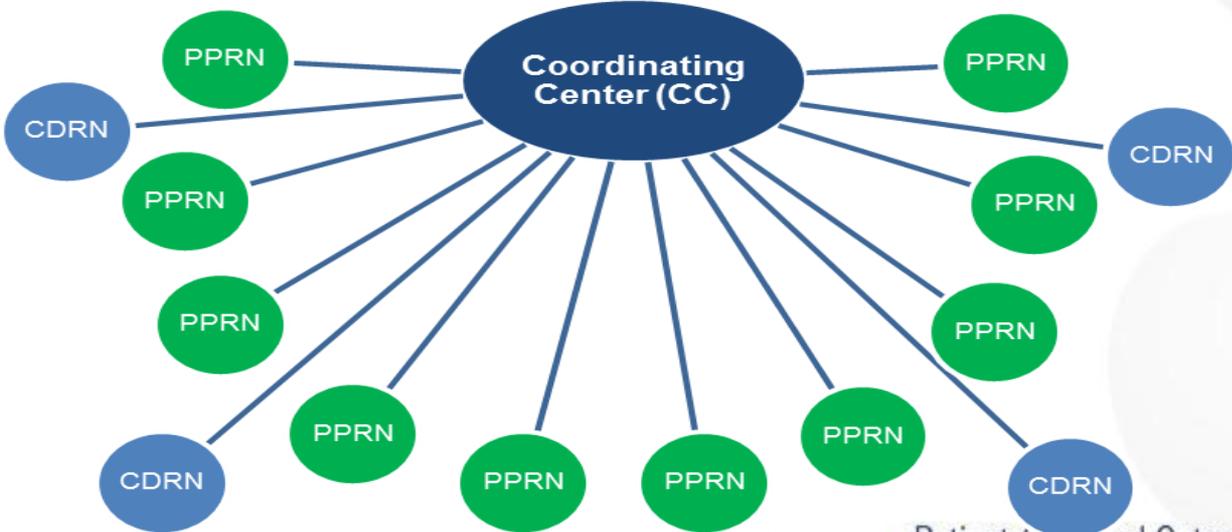


Steering Committee

Scientific Advisory Board

Special Expert Group

Coordinating Center Staff



Toward a National Patient-Centered Clinical Research Network



Potential Participants, Partners, Data Sources

Patient
Organizations

Medical
Groups

Disease
Registries

Practice Based
Research
Networks

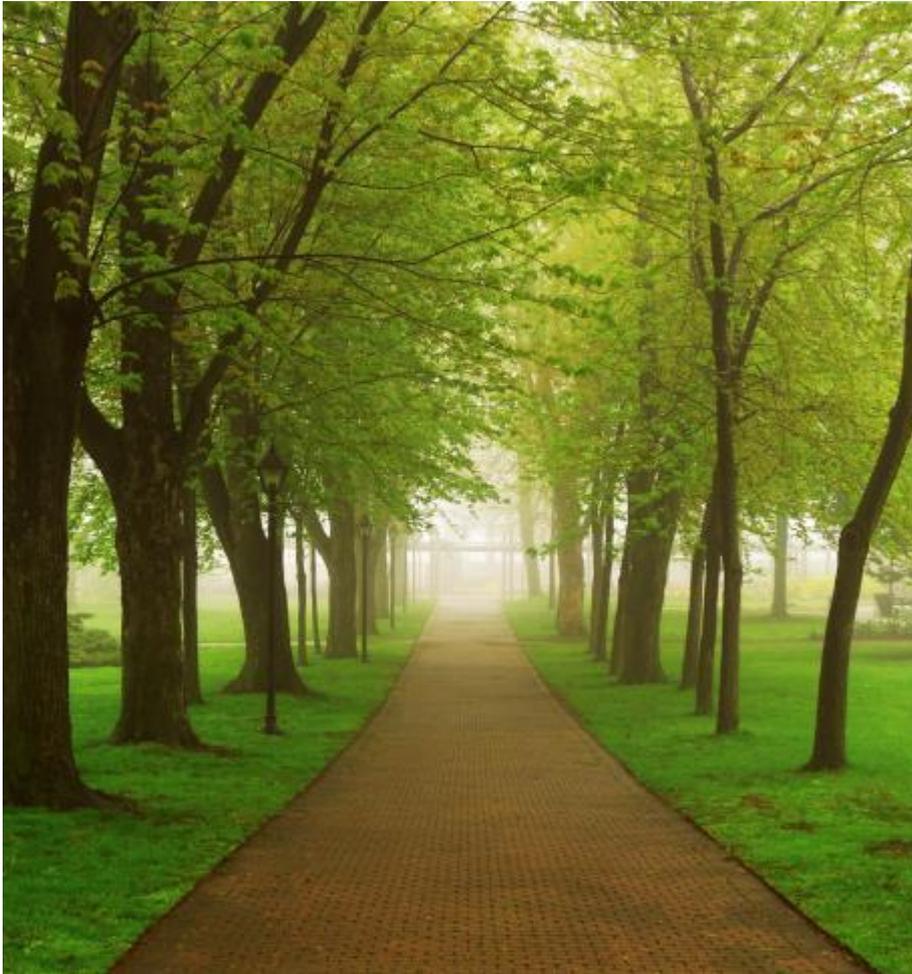
Integrated
Delivery
Systems

Health Plans

CMS, State,
County
Health Data

Medical
Centers

This is just the beginning...



Today's Roundtable

- What is the healthcare community's vision for how a national data network can support research and address the questions that matter most to patients?
- What are the challenges in creating a national research infrastructure?
- Where can PCORI's investments provide unique value?