Engaging Patients in Research and Tool Development

Joe V. Selby, MD, MPH, Executive Director

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Who We Are

An independent research organization authorized by Congress as part of the 2010 Patient Protection and Affordable Care Act.

We fund 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

Assessment of Prevention, Diagnosis, Treatment Options

• Comparisons of alternative clinical options; identifying patient differences in response to therapy; studies of patient preferences for various outcomes.

Improving Healthcare Systems

• Improving system support of patient self-management, coordination of care for complex conditions, access to care; comparing alternative strategies for workforce deployment.

Communication & Dissemination Research

• Understanding and enhancing shared decision-making; alternative strategies for dissemination of evidence; exploring opportunities to improve patient health literacy.

Addressing Disparities

• Understanding differences in effectiveness across groups; understanding differences in preferences across groups; reducing disparities through use of findings from PCOR.

Accelerating PCOR and Methodological Research

• Improving study designs and analytic methods of PCOR; building and improving clinical data networks.
Engagement: A Path To Rigorous, Useful Research

Advise Us on what PCORI Should Study:
• Topic generation
• Research prioritization

Review Proposals and Partner in Research
- Merit review
- Participate in research

Tell Us How We Are Doing
How can we improve on what we are doing and how we are doing it?

Help Us Share the Findings
- Dissemination

Patients and Stakeholders
Our Interest in Data and Tools

**Better Data**: Real-world clinical care; patient-reported information and outcomes.

**Better Infrastructure**: For faster collection, more efficient conduct of studies, and more reliable implementation in health care delivery.

**Better Decisions**: Practical tools that will harness the evidence to assist patients, their caregivers, and clinicians in making health care decisions.
Our Work in Decision-Support/Tool Development

- Shared decision-making, evidence-based decision-making is central to PCORI strategies for improving health.

- Many of PCORI’s Pilot Projects involve patients and clinicians in creating better tools for decision-making, tailored to specific patient populations.

- A number of our primary research projects also involve development of shared decision-making tools.
A BioScreen for Multiple Sclerosis

- **Primary Investigator and Institution:** Stephen Hauser, MD, University of California at San Francisco
- **Pilot Project Award Amount:** $599,966
- **Description:** Seeks to develop an effective and secure digital portal named BioScreen to access and display real-time clinical and biomarker information for use by patients and health providers. Focusing on multiple sclerosis, it will target three specific aims:
  - Integrate multiple dimensions of patient information in a single modular navigation system, including: clinical evolution of the disease, treatments, environmental exposures, brain imaging, plus genomic and biomarker data.
  - Enable the analysis of an individual's clinical course and data within the context of relevant reference groups, thus creating a transparent platform to quantify clinical outcomes and disease trajectories.
  - Facilitate the patient's engagement in disease management and data acquisition.

**Featured Project Strengths:**
- Develops a digital portal that can be extended to the treatment of a variety of chronic conditions.
- Combines the realms of personalized medicine, education, social media and telemedicine to improve patient care.
Integrating Patient-Centered Outcomes in Arthritis Clinical Care

**Primary Investigator and Institution:** Clifton O. Bingham, MD, The Johns Hopkins University (MD)

**Pilot Project Award Amount:** $655,944

**Description:** Will evaluate the impact of integrating enhanced patient-centered outcomes into clinical encounters, with rheumatoid arthritis as a model for chronic diseases. The Patient Reported Outcomes Measurement Information System (PROMIS) will be implemented to achieve these specific aims:

- Evaluate the integration of expanded PCOs using PROMIS into a busy clinical practice setting.
- Estimate the effect of integrating expanded PCOs on patient-care team interactions and sensitivity to detect change.
- Integrate a diverse group of stakeholders through all stages of this research, to inform study design, increase engagement, disseminate findings, and identify opportunities for further PCO research priorities in rheumatology and other chronic diseases.

**Featured Project Strength:**

- Places PROMIS into real-world setting, integrating PCOs into chronic disease management.
Engaging Stakeholders to Improve Depression Management in a Tribal Health System

- **Primary Investigator and Institution:** Renee Robinson, PharmD, Southcentral Foundation (AK)
- **Pilot Project Award Amount:** $604,301
- **Description:** Will develop, test, and refine a tool to support decision making within a tribally-owned health care organization, using depression as example condition. There are three specific aims:
  - Identify stakeholder preferences and needs that influence depression treatment decisions in Alaska Native and American Indian people.
  - Develop a tool to help translate and integrate evidence-based guidelines, and stakeholder preferences and needs into depression management decisions.
  - Determine impact of the stakeholder-centered tool on health, service utilization, and outcomes.

**Featured Project Strengths:**
- Studies an issue with high prevalence in an underserved community.
- The results will be applicable to other chronic health concerns, and other racially and ethnically diverse populations with persistent health disparities.
Our “Big Data” Idea: Making This Vision Real

We’ve proposed development of a National Patient-Centered Clinical Research Network

- Two funding announcements for up to $68 million.
  - Clinical Data Research Networks (CDRNs).
  - Patient-Powered Research Networks (PPRNs).

Key Dates:

- April 23: Funding Announcements Released.
- June 19: Letters of Intent (LOI) Due.
- September 27: Applications Due.
- December: Awards Announced.
What Would Such a Network Look Like?

**Characteristics:**

- A “network of networks.”
- Active involvement of health care systems, clinicians, and patients.
- A commitment to establishing interoperability and data sharing across networks and ready collaboration with the larger research community.

**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 CER, safety studies, surveillance, etiologic research, and potentially for pre-approval trials.
What the Ideal Data Network Will Do

Ideal Data Infrastructure for Patient-Centered CER

- Covers large, diverse, defined populations from usual care settings
- Allows for complete capture of longitudinal data
- Capacity for collecting patient reported outcomes
- Active patient and clinician engagement in governance of data use
- Is efficient in terms of costs for data acquisition, storage, analysis
- Linkages to health systems for rapid dissemination of findings
- Capable of randomization—at individual and cluster levels
National Patient-Centered Clinical Research Network

Patient Organizations
National Patient-Centered Clinical Research Network: Our Vision

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

Scientific Advisory Board

Special Expert Group

Coordinating Center Staff

Coordinating Center (CC)
Patient-Powered Research Networks (PPRNs)

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

**COOPERATIVE AGREEMENT AWARD**

- 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.

**18 MONTHS LATER**

- 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.
Clinical Data Research Networks (CDRNs)

$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.
Potential Participants, Partners, Data Sources

- Patient Organizations
- Medical Groups
- Disease Registries
- Practice Based Research Networks
- Integrated Delivery Systems
- Health Plans
- CMS
- State/County Health Systems
- Medical Centers
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