PCORI Funding Announcement 5B: Infrastructure

Rick Kuntz, MD, Member, PCORI Board of Governors,
Chair, Program Development Committee

PCORI Board of Governors Meeting
Washington, DC
September 25, 2012

pcori

Strategic Questions Around Infrastructure PFA

PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

1. Feedback on feasibility, opportunity for a "hybrid" approach to Infrastructure PFA
2. Strategic considerations for an Iterative, Progressive PFA Process

Two Distinct (and Complementary) Approaches Emerge from Palo Alto

PCORI National Workshop to Advance Use of Electronic Data

- Clinical Data Research Network (CDRN)
- Patient-Powered Research Network (PPRN)

Infrastructure Subgroup Is Formed

PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

Program Development Committee Members and Guests

- Carolyn Clancy
- Francis Collins
- Arnie Epstein
- Sherine Gabriel
- Christine Goertz
- Leah Hole-Curry
- Gail Hunt
- Harlan Krumholz
- Rick Kuntz
- Mike Lauer
- Nancy Miller
- Jean Slutsky

PCORI Executive Leadership and Staff

- Joe Selby
- Katie Wilson (Project Management)

An Iterative, Strategic Proposal for Funding

PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

Proposed Progression of PFAs

Phase One		Phase Two
PFA #1: Clinical Data Research Network	**PFA #2:** Patient-Powered Research Network	**PFA #3:** Patient-Centered Outcomes Research Network (PCORNet)
Answers Question: Can direct involvement of patients improve the efficiency and effectiveness of large data networks for conducting clinical research?	*Answers Question:* Can patient organizations and networks play a leading role in comparative effectiveness research?	*Builds on PFAs #1 & #2:* With a stable foundation for the conduct of many different research studies, now the projects need to be defined; may include coordinating/convening center award

Phase One: Clinical Data Research Network (1 of 3)

PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

Funding Opportunity

Invite consortia of multiple health services organizations to apply for support of research infrastructure:

- Goal #1: build large interoperable network(s) capable of supporting large scale comparative effectiveness trials of multiple research questions, including prevention and treatment, at low marginal cost, with substantive patient involvement throughout
- Goal #2: embed research activity within a functioning health care system without disrupting the business of providing health care; thus, business case for the health care system to participate must be addressed
- Goal #3: demonstrate ability to mobilize and engage substantial patient populations within the covered population for at least three conditions
- Goal #4: ensure health care organization leaders have meaningful role in governance, including a voice in identifying meaningful research priorities for their organization

Phase One: Clinical Data Research Network (2 of 3)

PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

Criteria

1. Patient, provider, and health system involvement at all levels of consortium design, implementation, governance, and use
2. Health care organization leaders must have a voice in research priorities (discouraging studies that are not feasible, identifying meaningful questions for their organizations)
3. Access to at least 1,000,000 patients
4. Potential for longitudinal follow up across care settings
5. Diversity in age, gender, socioeconomic status, race/ethnicity
6. Ability to obtain informed consent, using central IRB
7. Ability to establish at least three disease-specific cohorts that achieve participation of patients within the network, but also include outreach to established patient networks focused on those same disorders (see PFA #2)
8. Electronic health records with meaningful use for research
9. Buy-in and active participation from the health care system(s) in which care is delivered
10. Ability to conduct observational *and* interventional (individual or cluster randomized) trials
11. Data access policies that promote broad research use but protect privacy, confidentiality
12. Potential to obtain and store biological specimens

Phase One: Clinical Data Research Network (3 of 3)

PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

Allowable Items to Include in Proposal and Budget Request

- Permanent research staff support
- Incentives for the health system to support embedded research in the system
- Patient network outreach coordinator
- Communication staff
- Establishment of IT connectivity between consortium members
- Costs of consent for research
- Pilot research studies, ideally in partnership with relevant patient groups
- Biobank costs
- Patient surveys to assess interest in:
    - Generating research questions
    - Using the database
    - Ability to communicate with other patients
    - Providing patient-reported information
    - Participating in appropriate RCTs

Phase One: Patient-Powered Research Network (1 of 3)

PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

Funding Opportunity

Invite patient-centered organizations with specific disease or condition focus to apply for support:

- Goal: initially to provide pilot funding to help groups organize and develop the necessary infrastructure to define the most important patient-centered research questions and to prepare for vigorous, broad participation;
- Goal (latent objective): increase access to partnership and participation in research for a broadly diverse population of individuals with common health issues

Phase One: Patient-Powered Research Network (2 of 3)

PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

Criteria
1. Applicant group must demonstrate that they are considered credible and effective by those with the condition 2. Must represent a substantial number of affected individuals, ideally with diversity in demographics and disease severity, and with long term involvement of most participants, as appropriate given the condition 3. Group leadership must be representative in some way and be able to receive and act upon broad input of affected individuals, reflecting their needs and have processes in place to negotiate divergent opinions 4. Must have sufficient administrative and financial accounting structures to be able to receive and account for grant funds (PCORI staff may need to help here) 5. Must have interest in and capacity to expand membership to include historically under-represented patient groups with the condition

Phase One: Patient-Powered Research Network (3 of 3)

PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

Allowable Items to Include in Proposal and Budget Request

- Staff support to build and expand network of affected individuals, establish databases
- Administrative staff for grants management and grant preparation
- Infrastructure support to enhance interactive communication with patients and caregivers
- Funds for workshops to convene patients, clinicians, and other stakeholders, to identify the most important research questions
- Funds to address approaches to obtaining clinical electronic health record data on patients in the group
- Funds for outreach and enrollment of patients from traditionally under-representative populations
- Patient surveys to assess the demographic and clinical characteristics of enlisted patients and to study their interest in:
    - Generating research questions
    - Using the database
    - Ability to communicate with other patients
    - Providing patient-reported information
    - Participating in appropriate RCTs

Phase Two: Research Projects

Patient-Centered Outcomes Research Network

- **Disease-specific applications:**
  - Ideally come jointly from a PPRN applicant and a CDRN applicant
    - PPRN: Highly motivated, potentially less generalizable
    - CDRN: Less motivated, potentially more representative
  - Conditions not represented by a PPRN could be proposed with the CDRN Group to help catalyze such a group, starting with their own members, and extending more broadly
- **Outside investigators** could apply for the opportunity to run observational or interventional research studies through the CDRN and PPRN, ideally focused on conditions represented by the PPRN
- **Applications focused on prevention strategies** might need to come from an CDRN, or from an outside investigator proposing to have the CDRN conduct the study
- **Applications coming from health care organizations** ensure leaders have meaningful role in governance, including a voice in identifying meaningful research priorities for their organization

Next Steps

PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

- Finalize Hybrid Vision
- Conduct Inventory of Existing Patient Data Networks for Feasibility Planning, Taxonomy of Networks
- Release First Infrastructure PFA Q1, 2013

Strategic Questions Around Infrastructure PFA

PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

1. Feedback on feasibility, opportunity for a "hybrid" approach to Infrastructure PFA
2. Strategic considerations for an Iterative, Progressive PFA Process

Appendix

Strategic Document: PCORI Funding Opportunity 5b—Infrastructure for Research (document to be circulated)