PCORnet: The National Patient-Centered Clinical Research Network

A FasterCures Webinar
January 29, 2014
About FasterCures

- We are an **action tank** that works to improve and speed up the time it takes to get important new medicines from **discovery to patients**.

- We create opportunities and provide a platform for nontraditional allies to come together to share ideas and find partners.

- We work across diseases, sectors, and disciplines.

- We are a center of the Milken Institute.
• Loose affinity network of 60 nonprofit disease research foundations

• Created to tackle the challenges that cut across diseases through innovative partnerships

• Connected through TRAIN Central Station, an open-source web platform: www.fastercures.org/train
Finding Big Ideas in Small Spaces
Value and Coverage: How reimbursement decisions impact innovations needed to improve health

Introduction:
The U.S. healthcare system is in a period of dramatic change. As the imperative to control costs has become central, the concept of "value" is discussed everywhere, but its definition and the implications for medical research innovation are unclear. Pressure from payers on biopharma, medical devices, and diagnostics companies for "real-world evidence"—as opposed to the "gold standard" of randomized controlled trials required for U.S. Food and Drug Administration (FDA) approval—is growing. Companies are seeking new ways of showing value to payers and policymakers. Patient organizations are concerned that reimbursement decisions will be put far out of patients' reach. We need to materialize reimbursements issues aren't tackled.

On July 9, 2013, FasterCures and the Cystic Fibrosis Foundation (CF) convened a one-day workshop, "Value and measurement: What will the new day look like for patients?" More than 50 leaders from the FasterCures TRAINER (The Research Acceleration and Innovation Network: biotechnology and pharmaceutical companies, payers (public and private), policy-makers, and provider organizations) met in Washington, DC. Participants engaged in a candid discussion focused on defining "value" in cost-reimbursement decisions for life-saving therapies. They grappled with issues ranging from the evidentiary needs of payers to methods used to capture patient preferences—all toward the goal of protecting innovation in therapy development. The key themes that emerged throughout the day are reflected in this report.
Who’s logged on?

Attendees

- Researchers: 40%
- Advocacy Orgs: 30%
- Industry: 12%
- Patients/Consumers: 12%
- Training Institutions: 2%
- Other: 4%
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Speakers

Joe V. Selby
Executive Director,
Patient-Centered Outcomes Research Institute

Susan Sheridan
Director, Patient Engagement
Patient-Centered Outcomes Research Institute

John Walsh
Co-Founder & President,
COPD Foundation & PPRN awardee

Margaret Anderson
Executive Director,
FasterCures

MODERATOR
Introducing PCORnet: The National Patient-Centered Clinical Research Network

Joe Selby
Sue Sheridan

Presentation to TRAIN, FasterCures
January 29, 2014
PCORnet: the 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 clinical research more efficiently, by creating a large, highly representative, national patient-centered clinical research network with a focus on conducting CER – both randomized and observational.

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 within real-world care delivery systems.
Patient-centeredness is at the heart of PCORI’s name, and of its mission and vision

- 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 Emphasizes Engagement for Getting to Relevant, Useful Research

- Topic Generation and Prioritization
- Review and Conduct of Research
- Portfolio Review
- Dissemination and Implementation
Overall objectives of PCORnet: achieving a single functional research network

- Building a “network of networks” with inter-operable EHR, claims and patient-reported data on millions of individuals
- **Engagement** of patients, providers and health system leaders
- Support and conduct of multi-center observational and interventional CER studies
- **External data and research partners** participate with PCORI-funded networks
- Researchers not directly affiliated with PCORnet participate through collaborative arrangements.
- PCORnet partners **use the resources created with PCORI’s support** for a range of research activities supported by PCORI and other sponsors.
PCORnet Steering Committee

- Each Clinical Data Research Network (n=11)
- Each Patient Powered Research Network (n=18)
- Patient representative
- HHS agencies (potential funders, data suppliers)
  - NIH
  - FDA
  - AHRQ
  - CDC
  - CMS
  - ONC
  - ASPE
- Pharmaceutical / device manufacturers
- PCORI and Coordinating Center
29 CDRN and PPRN awards were approved on December 17th by PCORI’s Board of Governors

This map depicts the number of PCORI funded Patient-Powered or Clinical Data Research Networks that have coverage in each state.
Clinical Data Research Network Partners
Goals for Clinical Data Research Networks (CDRNs)

- Extracts from **EHR data** are standardized within PCORnet
- Complete, longitudinal clinical data on a minimum of **1 million patients** within 18 months
- Individual network’s data in **interoperable format** with other awardee networks and successfully responds to **queries**
- Willingness, capability of implementing **clinical trials**
- **Patients, health system leaders, and clinicians** involved in governance and use of network
- At least **three patient cohorts** identified, characterized, and surveyed
CDRN Highlights

- Networks of academic medical centers, hospitals and physician practices
- Networks of non-profit integrated health systems
- Networks of low income community-based clinics (FQHCs)
- Networks leveraging NIH and AHRQ investments (CTSAs)
- Inclusion of Health Information Exchanges
- Wide geographical spread
- Inclusion of underserved populations
- Range from 1M covered lives to 28M
<table>
<thead>
<tr>
<th>CDRN Name</th>
<th>Lead Organization</th>
<th>Principal Investigator</th>
</tr>
</thead>
<tbody>
<tr>
<td>ADVANCE</td>
<td>Oregon Community Health Information Network</td>
<td>Jennifer DeVoe</td>
</tr>
<tr>
<td>CAPriCORN</td>
<td>The Chicago Community Trust</td>
<td>Terry Mazany</td>
</tr>
<tr>
<td>Great Plains Collaborative</td>
<td>University of Kansas Medical Center</td>
<td>Lemuel Waitman</td>
</tr>
<tr>
<td>Louisiana Clinical Data Research Network</td>
<td>Louisiana Public Health Institute</td>
<td>Anjum Khurshid</td>
</tr>
<tr>
<td>Mid-South CDRN</td>
<td>Vanderbilt University</td>
<td>Russell Rothman</td>
</tr>
<tr>
<td>NYC-CDRN</td>
<td>Weill Medical College of Cornell University</td>
<td>Rainu Kaushal</td>
</tr>
<tr>
<td>PEDSNet</td>
<td>The Children’s Hospital of Philadelphia</td>
<td>Christopher Forrest</td>
</tr>
<tr>
<td>PORTAL</td>
<td>Kaiser Foundation Research Institute</td>
<td>Elizabeth McGlynn</td>
</tr>
<tr>
<td>pSCANNER</td>
<td>University of California, San Diego</td>
<td>Lucila Ohno-Machado</td>
</tr>
<tr>
<td>P2ATH</td>
<td>University of Pittsburgh</td>
<td>Rachel Hess</td>
</tr>
<tr>
<td>SCIHLS</td>
<td>Harvard University</td>
<td>Kenneth Mandl</td>
</tr>
</tbody>
</table>
## CDRNs Disease Cohorts

<table>
<thead>
<tr>
<th>Organization</th>
<th>Common Cohort</th>
<th>Rare Cohort</th>
</tr>
</thead>
<tbody>
<tr>
<td>ADVANCE</td>
<td>Diabetes</td>
<td>Co-infection with HIV and hepatitis C virus</td>
</tr>
<tr>
<td>CAPriCORN</td>
<td>Anemia; Asthma</td>
<td>Sickle cell disease; Recurrent C. Difficile colitis</td>
</tr>
<tr>
<td>Great Plains Collaborative</td>
<td>Breast Cancer</td>
<td>Amyotrophic Lateral Sclerosis (ALS)</td>
</tr>
<tr>
<td>Louisiana Clinical Data Research Network</td>
<td>Diabetes</td>
<td>Sickle Cell Disease, Rare Cancers</td>
</tr>
<tr>
<td>NYC-CDRN</td>
<td>Diabetes</td>
<td>Cystic fibrosis</td>
</tr>
<tr>
<td>Mid-South CDRN</td>
<td>Coronary Heart Disease (CHD)</td>
<td>Sickle Cell Disease (SCD)</td>
</tr>
<tr>
<td>PEDSNet</td>
<td>Inflammatory bowel disease</td>
<td>Hypoplastic left heart syndrome</td>
</tr>
<tr>
<td>PORTAL</td>
<td>Colorectal Cancer</td>
<td>Severe Congenital Heart Disease</td>
</tr>
<tr>
<td>pSCANNER</td>
<td>Congestive Heart Failure</td>
<td>Kawasaki Disease</td>
</tr>
<tr>
<td>P2ATH</td>
<td>Atrial Fibrillation</td>
<td>Idiopathic Pulmonary Fibrosis</td>
</tr>
<tr>
<td>SCIHLS</td>
<td>Osteoarthritis</td>
<td>Pulmonary arterial hypertension</td>
</tr>
</tbody>
</table>
Patient-Powered Research Network Partners
Goals for Patient-Powered Research Networks (PPRNs)

- Target size of **0.5% of U.S population** with condition: (> 50 patients for rarest diseases; 50,000 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 and successfully responds to queries
PPRN Highlights

- 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.)
- 50% rare diseases
- Significant range in the maturity of the group in terms of data available
- Several have capacity to work with biospecimens
PPRNs represent a number of conditions…

<table>
<thead>
<tr>
<th>Organization</th>
<th>PI</th>
<th>Condition</th>
<th>Proposed PPRN Population Size</th>
</tr>
</thead>
<tbody>
<tr>
<td>Accelerated Cure Project for Multiple Sclerosis</td>
<td>Robert McBurney</td>
<td>Multiple Sclerosis</td>
<td>20,000</td>
</tr>
<tr>
<td>American Sleep Apnea Association</td>
<td>Susan Redline</td>
<td>Sleep Apnea</td>
<td>50,000</td>
</tr>
<tr>
<td>Cincinnati Children's Hospital Medical Center</td>
<td>Peter Margolis</td>
<td>Pediatric Crohn's Disease and Ulcerative Colitis</td>
<td>15,000</td>
</tr>
<tr>
<td>COPD Foundation</td>
<td>Richard Mularski</td>
<td>Chronic Obstructive Pulmonary Disease</td>
<td>50,000</td>
</tr>
<tr>
<td>Crohn’s and Colitis Foundation of America</td>
<td>R. Balfour Sartor</td>
<td>Inflammatory Bowel Disease (Crohn’s disease and ulcerative colitis)</td>
<td>30,000</td>
</tr>
<tr>
<td>Global Healthy Living Foundation</td>
<td>Seth Ginsberg</td>
<td>Arthritis (rheumatoid arthritis, spondyloarthritis), musculoskeletal</td>
<td>50,000</td>
</tr>
<tr>
<td></td>
<td></td>
<td>disorders (osteoarthritis), and inflammatory conditions (psoriasis)</td>
<td></td>
</tr>
<tr>
<td>Massachusetts General Hospital</td>
<td>Andrew Nierenberg</td>
<td>Major Depressive Disorder (MDD) and Bipolar Disorder (BP)</td>
<td>50,000</td>
</tr>
<tr>
<td>Univ of California, San Francisco</td>
<td>Mark Pletcher</td>
<td>Cardiovascular health</td>
<td>100,000</td>
</tr>
<tr>
<td>University of South Florida</td>
<td>Rebecca Sutphen</td>
<td>Hereditary Breast and Ovarian Cancer (HBOC)</td>
<td>17,000</td>
</tr>
</tbody>
</table>
including rare diseases

<table>
<thead>
<tr>
<th>Organization</th>
<th>PI</th>
<th>Condition</th>
<th>Proposed PPRN Population Size</th>
</tr>
</thead>
<tbody>
<tr>
<td>ALD Connect, Inc</td>
<td>Florian Eichler</td>
<td>Adrenoleukodystrophy</td>
<td>3,000</td>
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<tr>
<td>Arbor Research Collaborative for Health</td>
<td>Bruce Robinson</td>
<td>Primary Nephrotic Syndrome (Focal Segmental Glomerulosclerosis [FSGS], Minimal Change Disease [MCD], and Membranous Nephropathy [MN] Multiple Sclerosis</td>
<td>1,250</td>
</tr>
<tr>
<td>Duke University</td>
<td>Laura Schanberg</td>
<td>Juvenile Rheumatic Disease</td>
<td>9,000</td>
</tr>
<tr>
<td>Epilepsy Foundation</td>
<td>Janice Beulow</td>
<td>Aicardi Syndrome, Lennox-Gastaut Syndrome, Phelan-McDermid Syndrome, Hypothalamic Hamartoma, Dravet Syndrome, and Tuberous Sclerosis</td>
<td>1,500</td>
</tr>
<tr>
<td>Genetic Alliance, Inc</td>
<td>Sharon Terry</td>
<td>Alström syndrome , Dyskeratosis congenital, Gaucher disease, Hepatitis, Inflammatory breast cancer, Joubert syndrome, Klinefelter syndrome and associated conditions, Metachromatic leukodystrophy, Pseudoxanthoma elasticum (PXE), Psoriasis</td>
<td>50- 50,000</td>
</tr>
<tr>
<td>Immune Deficiency Foundation</td>
<td>Kathleen Sullivan</td>
<td>Primary Immunodeficiency Diseases</td>
<td>1,250</td>
</tr>
<tr>
<td>Parent Project Muscular Dystrophy</td>
<td>Holly Peay</td>
<td>Duchenne and Becker muscular dystrophy</td>
<td>4,000</td>
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<tr>
<td>Phelan-McDermid Syndrome Foundation</td>
<td>Megan O’Boyle</td>
<td>Phelan-McDermid Syndrome</td>
<td>737</td>
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<tr>
<td>University of Pennsylvania</td>
<td>Peter Merkel</td>
<td>Vasculitis</td>
<td>25</td>
</tr>
</tbody>
</table>

Population Size
Patient Engagement in Infrastructure Development in the PPRN

Enrollment and Diversity
- Increasing size of the network
- Increasing the diversity of the network
- Retention of network members

Governance
- The development of the network governance structure, roles and responsibilities
- Development of procedures, bylaws and policies for the network

Data collection
- The development of data collection tools
- Identification of Patient Reported Outcomes (PROs) for inclusion in database

Data sharing and privacy
- The development of consent processes and policies
- Development of data sharing agreements
Coordinating Center Co-Directors

Richard Platt, MD, MSc

Robert M. Califf, MD
CC Executive Leadership Committee

Richard Platt, Harvard Pilgrim Health Care Institute, Harvard Medical School

Robert Califf, Director, Duke Translational Medicine Institute, Vice Chancellor for Clinical and Translational Research

Eric Larson, Group Health Research Institute

Mark McClellan, Brookings Institution

Erin Holve, AcademyHealth

Bray Patrick-Lake, Director of Stakeholder Management, Duke Translational Medicine Institute
Multiple Networks Sharing Infrastructure

- Each organization can participate in multiple networks
- Each network controls its governance and coordination
- Networks share infrastructure, data curation, analytics, lessons, security, software development
Some takeaways

We need a new national capability for **efficient, large-scale interventional and observational patient-centered research**

PCORnet will need to:

- Establish **priorities** that patients, clinicians, clinical leaders, and investigators share
- Facilitate **trust** leading to collaboration between networks
- Embed research into **practice settings** without disrupting clinical operations
- Create a **distributed data network** that protects patients’ **confidential** information
- Develop **oversight** procedures that protect patients while minimizing redundancy
- Engage **individuals and organizations** beyond the initial awardees
John Walsh

COPD Foundation Co-founder and President
Joe V. Selby  
*Executive Director, Patient-Centered Outcomes Research Institute*

Susan Sheridan  
*Director, Patient Engagement Patient-Centered Outcomes Research Institute*

John Walsh  
*Co-Founder & President, COPD Foundation & PPRN awardee*

Margaret Anderson  
*Executive Director, FasterCures*
View an archive of this Webinar
www.fastercures.org/train
Save the Date!
Next month’s TRAIN Webinar

FDA’s Patient-Focused Drug Development Initiative and the Benefit-Risk Assessment Framework

Wednesday, February 19, 2014
1-2 PM Eastern