Current State of PCORnet, Clinical Data Research Networks, and the Greater Plains Collaborative

Russ Waitman, Steve Fennel, Jim McClay

Greater Plains Collaborative Learning Engagement Conference

October 13, 2016

PCORnet® The National Patient-Centered Clinical Research Network
Want to share:

- Recent Overview of the network by PCORI from August

- The latest update from the national PCORnet meeting last week
  - PCORnet Sustainability
  - ADAPTABLE trial as our demonstration project

- “Robert Califf inspired” observations on network development
  - Russ sits on the executive committee now as he’s the data committee chair for PCORnet

- **Your charge:** help us consider the best path ahead for you, your “site”, your regional partners, the GPC, and PCORnet.
  - Provide feedback as we evaluate the next several months over calls and meetings
Russ’ Executive Summary

PCORnet is very complex and ambitious. We have
- Integrated billing+clinical data at enormous scale and now claims
- Heightened coordination between regulatory and informatics teams to support our researchers
- Researchers and patients are coming together to improve research

But… we and the national network are behind schedule
- Conducting massive studies as the infrastructure is developing reveals obstacles/misalignment but also frays relationships
- “Everything is amazing and nobody is happy”

PCORI stated goal: transition PCORnet from a network bound by contracts with them to independence

Concern: The current approach, pace, and investment in data infrastructure (eg. MiniSentinel for FDA versus many customers) may not optimally align with sustainability

How do we position the GPC and our sites for future success?
Overview of PCORnet: The National Patient-Centered Clinical Research Network

Maryan Zirkle, MD, MS, MA

Program Officer, Research Infrastructure, PCORI

August 8, 2016
PCORnet is a national patient-centered clinical research network developed by the nonprofit Patient-Centered Outcomes Research Institute (PCORI). PCORnet seeks to improve the nation’s capacity to conduct clinical research by creating a large, highly representative, national patient-centered network that supports more efficient clinical trials and observational studies.
PCORnet
$300+ Million Infrastructure Investment

- January 2014 : Phase I
  - 11 CDRNs
  - 18 PPRNs
  - Coordinating Center

- August 2015: Governance in place

- October 2015: Phase II begins
  - 13 CDRNs
  - 20 PPRNs
  - Coordinating Center
PCORnet Organizational Readiness: Structure and Decision-making

Role Descriptions:

Executive Committee (EC): Oversight for PCORnet operations

PCORnet Council: Representative governing body for PCORnet strategy and operations; includes PCORnet’s 13 Clinical Data Research Networks (CDRNs) and 20 Patient-Powered Research Networks (PPRNs)

Coordinating Center (CC): Coordinates operational activities, maintains data infrastructure, identifies research opportunities, coordinates multi-site research
PCORnet Coordinating Center (CC)

Leads PCORnet operations and data activities, and supports PCORnet infrastructure

Responsibilities:
- Coordinate PCORnet’s operations
- Lead development and maintenance of PCORnet’s shared data infrastructure
- Identify opportunities for PCORnet research
- Support multi-site research
- Engage with networks to facilitate support and transparency

Participating CC organizations:
- Duke Clinical Research Institute (DCRI),
- Harvard Pilgrim Health Care Institute (HPHCI), and
- Genetic Alliance, Inc.
Executive Committee-Oversight for PCORnet Operations

- **Joe V. Selby**, MD, MPH, PCORI Executive Director
- **Rachael Fleurence**, PhD, PCORI, PCORnet Program Director
- **Adrian Hernandez**, MD, MHS, Co-Principal Investigator, PCORnet Coordinating Center (DCRI)
- **Peter Margolis**, MD, PhD, Chair, Executive Committee & Principal Investigator ImproveCareNow PPRN
- **Elizabeth McGlynn**, PhD, Principal Investigator, Kaiser Permanente & Strategic Partners Patient Outcomes Research To Advance Learning (PORTAL) Network
- **Peter A. Merkel**, MD, MPH, Principal Investigator, The Vasculitis Patient Powered Research Network
- **Richard Platt**, MD, MSc, Co-Principal Investigator, PCORnet Coordinating Center (HPHCI)
- **Russell Rothman**, MD, Principal Investigator, Mid-South CDRN
- *Sharon Terry*, MA, Principal Investigator, PCORnet Coordinating Center (GA) & PI, Community-Engaged Network for All (CENA)
- *Christopher B. Forrest*, MD, PhD, Principal Investigator, PEDSnet: A Pediatric Learning Health System
- *Russ Waitman*, PhD, Principal Investigator, Greater Plains Collaborative (GPC)

*indicates Committee Chairs, non-voting members of the EC
PCORnet embodies a “community of research” by uniting people, clinicians & systems

20 Patient-Powered Research Networks (PPRNs) + 13 Clinical Data Research Networks (CDRNs) = PCORnet

A national infrastructure for people-centered clinical research
CDRN highlights

- Networks of academic health centers, hospitals & clinical practices
- Networks of non-profit integrated health systems
- Networks of Federally Qualified Health Centers (FQHCs) serving low-income communities
- Networks leveraging NIH and AHRQ investments (CTSAs)
- Inclusion of Health Information Exchanges
- Wide geographical spread
- Inclusion of under-served populations
- Range from 1M covered lives to 28M
PCORnet Clinical Data Research Networks (CDRNs) – Phase II

- The Chicago Community Trust (CAPriCORN)
- The Children’s Hospital of Philadelphia (PEDSnet)
- Harvard University (SCILHS)
- Kaiser Foundation Research Institute (PORTAL)
- Louisiana Public Health Institute (REACHnet)
- Mayo Clinic (LHSNet)
- Oregon Community Health Information Network (ADVANCE)
- University of California, San Diego (pSCANNER)
- University of Florida (OneFLorida)
- University of Kansas Medical Center (GPC)
- University of Pittsburgh (PaTH)
- Vanderbilt University (Mid-South CDRN)
- Weill Medical College of Cornell University (NYC-CDRN)
PPRN highlights

- Participating organizations and leadership teams include patients, advocacy groups, clinicians, academic centers, practice-based research networks

- Strong understanding of patient engagement

- PPRNs represent different models of partnerships and levels of infrastructure, represent over 100 diseases overall
  - Approximately 50% are focused on rare diseases
  - Phase II brought in two community focused PPRNs

- Variety in populations represented (including children and under-served communities)

- Varying capabilities with respect to developing research data
PCORnet Patient-Powered Research Networks – Phase II

- University of South Florida (ABOUT Network)
- Global Health Living Foundation (AR-PoWER)
- Mayo Clinic (AD PCPRN)
- Crohn’s and Colitis Foundation of America (CCFA Partners)
- University of California Los Angeles (CPPRN)
- Genetic Alliance (CENA)
- COPD Foundation (COPD PPRN)
- Parent Project Muscular Dystrophy (DuchenneConnect)
- University of California San Francisco (Health eHeart Alliance)

- Cincinnati Children’s Hospital Medical Center (ImproveCareNow)
- Kennedy Krieger Institute (IAN)
- Massachusetts General Hospital (MOOD)
- Accelerated Cure Project for Multiple Sclerosis (MS-PPRN)
- Arbor Research Collaborative for Health (NephCure)
- Duke University (PARTNERS)
- Phelan-McDermid Syndrome Foundation (PMS_DN)
- Immune Deficiency Foundation (PI-CONNECT)
- University of California San Francisco (PRIDEnet)
- Epilepsy Foundation (REN)
- University of Pennsylvania (The Vasculitis PPRN)
Data Infrastructure
PCORnet is Growing

130+ health systems across the country

Over 80 DataMarts

Data on over 90 million individuals
Research-Ready PCORnet

Data Mart Totals:
March 31: 71
April 14: 75
May 12: 82
May 19: 83
May 26: 83
June 2: 84
June 29: 82

Data Characterization Progress

Approved for Research
Data Characterization Review
Prep-to-Research Ready Phase
Data Characterization Phase
Diagnostic Query Phase

Number of DataMarts

Mar 31 Apr 15 May 5 May 19 May 26 June 2 8-Aug

Time
Standardize to a common data model

**Fundamental basis**
- **DEMOGRAPHIC**
  Records direct attributes of individual patients.

**Data captured from processes associated with healthcare delivery**
- **ENROLLMENT**
  Concept that defines a period of time during which all medically-attended events are expected to be observed.

**Data captured within multiple contexts: healthcare delivery, registry activities, or directly from patients**
- **VITAL**
  Vital signs directly measure an individual’s current state of attributes.

**Data captured from healthcare delivery, direct encounter basis**
- **ENCOUNTER**
  Interactions between patients and providers within context of healthcare delivery.

- **DIAGNOSIS**
  Diagnosis codes indicate results of diagnostic processes and medical coding.

- **PROCEDURES**
  Procedure codes indicate discrete medical interventions or diagnostic testing.

- **PRESCRIBING**
  Provider orders for medication dispensing or administration.

- **LAB RESULT CM**
  Specific types of quantitative and qualitative measurements from blood and body specimens, standardized across all networks.

**Associations with PCORnet clinical trials**
- **PCORNET TRIAL**
  Patients who are enrolled in PCORnet clinical trials.

**Process-related data**
- **HARVEST**
  Attributes associated with specific PCORnet datamart implementations.

- **DEATH**
  Reported mortality information for patients.

- **DEATH CAUSE**
  Individual causes associated with a reported death.

- **PRO CM**
  Patient-Reported Outcome (PRO) Common Measures (CM) are standardized measures that are defined the same way across all PCORnet networks. Each measure is recorded at the individual item level.
Additional Linkage for “Complete” Data

- Death Index
- Condition
- Biospecimen & Genomic Data
- Procedures
- Encounters
- Lab Results
- Claims
- Demographic
- Prescribing
- Patient Satisfaction

pcornet®
Here’s how PCORnet’s distributed research network works

The Researcher sends a question to the PCORnet Coordinating Center through the Front Door.

The Coordinating Center converts the question into a query with an underlying executable code, and sends it to PCORnet partners.

PCORnet partners review the query and provide a response, which is sent back through the Front Door to the Researcher.
Research Developments
<table>
<thead>
<tr>
<th>Project Title</th>
<th>Award Status</th>
<th>Purpose</th>
<th>Number of Studies</th>
</tr>
</thead>
<tbody>
<tr>
<td>ADAPTABLE Clinical Trial</td>
<td>Awarded</td>
<td>Comparative effectiveness of 81 vs 325 mg of aspirin for secondary prevention of cardiac events and serious bleeding</td>
<td>1</td>
</tr>
<tr>
<td>Antibiotics and Excess Weight Gain in Children Observational Study</td>
<td>Awarded</td>
<td>To evaluate the comparative effects of different types, timing, and amount of antibiotics prescribed during the first 2 years of life and how these effects differ based on certain characteristics</td>
<td>1</td>
</tr>
<tr>
<td>Bariatric Surgery Observational Study</td>
<td>Awarded</td>
<td>Provide accurate estimates of the 1-, 3-, and 5-year benefits and risks of the three main surgical treatment options for severe obesity</td>
<td>1</td>
</tr>
<tr>
<td>PPRN Demonstration Projects</td>
<td>Awarded</td>
<td>Various PPRN-initiated research questions that have been generated and prioritized by participants within the PPRN community</td>
<td>5</td>
</tr>
<tr>
<td>Cross-PPRN Demonstration Project</td>
<td>Awarded</td>
<td>Comparative effectiveness project that will demonstrate scientific, administrative, and operational capacity to collaborate across PPRNs</td>
<td>1</td>
</tr>
<tr>
<td>Health Systems Demonstration Project</td>
<td>Awarded</td>
<td>Provide CDRNs the opportunity to test their capacity to conduct collaborative research with health systems leaders across PCORnet</td>
<td>4</td>
</tr>
</tbody>
</table>
## Data Linkage Projects

<table>
<thead>
<tr>
<th>Project Title</th>
<th>Award Status</th>
<th>Purpose</th>
<th>Number of Studies</th>
</tr>
</thead>
<tbody>
<tr>
<td>CMS Pilot Project</td>
<td>Awarded</td>
<td>To develop mechanism to link CMS data with CDRN EHR data</td>
<td>1</td>
</tr>
<tr>
<td>Health Plans</td>
<td>Awarded</td>
<td>To develop and test mechanisms for building more complete data by linking health plans claims data to EHR data</td>
<td>2</td>
</tr>
</tbody>
</table>
Obesity Observational Demonstration Studies

- Studies focused on the treatment and prevention of obesity in children and adults
  - (1) *Short- and Long-term Effects of Antibiotics on Childhood Growth*
  - (2) *PCORnet Bariatric Study*

- Retrospective observation studies that will test PCORnet’s Data Infrastructure:
  - **Functionalties of the Coordinating Center** and Network operations
  - Capacity for using **PCORnet’s Distributed Research Network** for answering important observational research questions
  - PCORnet’s ability to use **analysis-ready distributed datasets**
  - Ability to **run analytical SAS code** on completed datasets

- Studies began February 2016 and are 2 years in duration
Topics from PCORnet PI Retreat October 5th

- Landscape for PCORnet Sustainability
- Roadmap for establishing a PCORnet entity
- ADAPTABLE
Outsourced Research & Development market 2015

- Global Biopharmaceutical Sales ➕ $743B
- Global Biopharmaceutical R&D ➕ $142B
- Development Spending (Late discovery – PIV) ➕ $114B
- Core Addressable Market ➕ $47B*
- CRO Market ➕ $26B
- Early Dev ➕ $8B
- Late Stage Dev ➕ $14B
- Central Labs & eClinical ➕ $4B

Projected 3-year CAGR 6%

*excludes 20% for overhead, 20% for CMC activities and 10% for pass-through costs
NIH Spending & Budget Estimates

NIH Strategic Objectives

- Science of science
- Outputs, outcomes
- Workforce analyses
- Review peer review
- Rigor, reproducibility
- Administrative burden
- Risk management

NIH Focus on Quality and Efficiency

Improving Clinical Trials

**eFigure. Improving Clinical Trials.** The new, multifaceted effort shown above will enhance the quality and efficiency of NIH-supported clinical trials by focusing on a variety of key points along the “lifespan” of a clinical trial.

Hudson K et al JAMA. 2016 Sep 16
<table>
<thead>
<tr>
<th>Phase</th>
<th>Discovery &amp; Preclinical</th>
<th>Phase I-IIa Early Clinical</th>
<th>Bio-analytical</th>
<th>Phase IIb-IV Global Clinical</th>
<th>Central Labs</th>
<th>Other</th>
<th>eClinical</th>
</tr>
</thead>
<tbody>
<tr>
<td>Early Stage testing:</td>
<td>Determine impact of compound on human subjects (healthy or specific condition)</td>
<td>Analyzing samples from preclinical or early clinical to test for specific physiological impacts</td>
<td>Managing patient recruitment and administration of clinical trials for new compounds</td>
<td>Analyzing human samples from clinical trials to test for specific physiological impacts</td>
<td>Includes preclinical and clinical supplies, research models, formulation, and manufacturing</td>
<td>Includes electronic data capture, IVRS, RTSM, medical imaging</td>
<td></td>
</tr>
</tbody>
</table>

| Addressable Market | $8.5B | $6.8B | $1.0B | $36.9B | $2.3B | $10B | $2.3B |
| Outsourcing Penetration | 44% | 70% | 40% | 50% | 100% | 50% | 80% |
| CRO Market | $3.7B | $4.8B | $0.4B | $18.5B | $2.3B | $5B | $1.8 |
| Expected Market Growth | 6-8% | 3-5% | 6-7% | 6-8% | 4-6% | 4-6% | 4-6% |

**Key Players**
- Discovery & Preclinical: CVD, CRL
- Phase I-IIa Early Clinical: Celerion, Novum, Covance
- Bio-analytical: PharmaNet, CVD, PPD
- Phase IIb-IV Global Clinical: Quintiles, PPD, PRXL
- Central Labs: CVD, Quintiles
- Other: CRL, Sigma Aldrich, Thermo Fisher
- eClinical: Oracle, Medidata, PRXL

Core addressable market excludes 20% for overhead, 20% for Chemistry, Manufacturing, and Control (CMC) activities, and 10% for pass-through costs.
Competitive Landscape:

- Scalability of Network
  - Low
    - Multiple Myeloma Research Consortium
  - High
    - BD2K Initiative
    - High Value Healthcare Collaborative
    - GA4GH

- Integration of Data
  - Low
    - 23 and Me
    - Health Care Cost Initiative (HCCI) MaRS EXCITE
    - NIH Collaboratory NIH BTRIS
    - TriNetX
    - Transcelerat
    - NHS Clinical Research Initiative
  - High
    - Explorys
    - OptumLabs
    - PCORnet
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collaboratory NIH BTRIS
    - CancerLinQ CallINDEX
    - NIH Collaboratory NIH BTRIS
    - Health Catalyst
    - NIH Collab
Differentiation of PCORnet

- **Depth, breadth and engagement of thought leadership in clinical domains**
  - PPRNs/CDRNs
  - Collaborative research groups

- **Patient-focused research agenda**
  - Addressing specific needs of society

- **Differentiated, functioning, and evolving Common Data Model**
  - Access to validated clinical data
  - Up-to-date national level data
  - Quality and usability metrics
  - “Speed of” execution of queries

- **Network of institutions and organizations**
  - Clinical and patient-reported outcomes data marts

- **Real World Evidence Development**
  - Observational
  - Pragmatic Clinical Trials
  - Health System Research

- **Program office which coordinate and execute studies**
  - Large and small studies for both industry, non-profits, and federal government

- **Cost-efficient infrastructure**

- **Dissemination & Implementation**
Approach to PCORnet sustainability

Primary goal: Build a sustainable “Better, Faster and Cheaper platform with scale for patient-centered clinical research”

- Understand strategic needs, operational needs, and concerns and considerations as seen by Executive Committee
- Assess costs for ongoing operations and data network
- Develop potential revenue sources for sustainability
- Evaluate pros/cons of model and refine to fit for network members and potential users
- Develop overall business and operating plan
GOAL(s)

- **Administrative:**
  - Simplicity program
  - Establish new entity in 2017 and minimal infrastructure for new entity

- **Operational:**
  - Establish proof of concepts

- **Business development:**
  - Establish a pipeline of research and early adopters
Next Steps: To establish a PCORnet entity

- Completion of the full business plan
- Socialization of the business plan:
  - PCORnet members
  - Stakeholders
  - PCORI board
- Committed PCORnet members
  - To construct a governance model
  - To develop an operational plan
  - To refine, finalize, support the business plan
- Develop and finalize on-boarding plan for CDRN and PPRN
Next Steps

- Open the front door!
- Break down those walls!
- Develop *Early Adopters*
- Generate success
- Expand capacity & scale
Operating overview

CDRNs/PPRNs
1
2
13

COORDINATING CENTER
Lean office: program development coordinating center
• Matchmaking/networking services
• Data network ops
• Business development
• SOP for contacting and operations
• Pricing models
• Project management

PCORnet ENTITY
Non-Profit Corporation 501(c)(3)
• Administration
• Finance
• Marketing
• Business Dev
• Governance model
• Advisory Board

Research Market
• govt. agencies
• pcori
• industry
• non-profits
PCORnet engagement entry options

01 FRONT DOOR

Main double door to coordinate and execute studies sponsored by Government, FDA, NIH, Non-profits and Industry
Full-service suite from business development to contracting to coordination

02 TRANSACTION WINDOWS @CDRN/PPRN

Other entry points at subnet level for research studies
• specialty populations
• narrow network (geo / size)
• narrow thematic focus areas
Limited coordinating functions
Engagement through Front Door or Transaction Window

Note: Subnet-level coordinating centers may exist at some CDRNs or PPRNs to support research operations.
PCORnet for many kinds of research

Pre-research
- Feasibility queries
- Engagement
- Match-making

Interventional studies
- Clinical trials
- Pragmatic randomized clinical trials
  - e-Identification
  - e-Consent
  - e-Randomization
  - e-Follow-up
- Cluster randomization

Observational studies
- Cross-sectional
- Epidemiology
- Health services
- Comparative effectiveness or safety
Success Builds Success: Addressing Network Challenges

Adrian Hernandez

pcornet® The National Patient-Centered Clinical Research Network
Matching National Research
Top 5 Needs with PCORnet

Phase I

- Engaged clinical organizations and patients
- Collaboration framework
- Analysis-ready standardized data with strong privacy protections and ability for re-useable tools

Phase II

- Administrative simplicity with regulatory oversight that protects patients without unnecessary burdens
- Embed research in care settings
BENDING THE CURVE on ADAPTABLE

Turn the Curve Analysis:

**Optimization of the Protocol:** 2=3X eligible population by matching the I/E criteria with what’s available in the datamarts

**Go-Large & Multi-touch:** e-Contact, tele-contact, PBA, socialization
Goals for ADAPTABLE

Start-up/Administrative
- Engagement, Co-Creation
- IRB
- Contract

Operations
- Data
- E-Identification
- E-Recruitment
- E-Follow-up
Start-up Metrics - Adaptable vs Traditional

ADAPTABLE Metrics

- CDRN Contract Executed: Adaptable 132, Traditional 122
- IRB Approval: Adaptable 122, Traditional 133
- Initial CDRN Site Activation: Adaptable 189, Traditional 167
- Site Activation: Adaptable 239, Traditional 167

Data Current as of 9/28/2016
**Administrative Evaluation**

**Contracts:**
- Administration via Network ➔ Subcontract ≠ Efficient
  - Sub-contract negotiations escalates to prime
  - CDRNs are typically not legal entities that can ‘sign-off’ on contracts
- Master templates directed at site level offers potential

**IRB**
- IRB barriers increased with patient-facing materials
- CDRN reliance agreements vary widely
- sIRB (SMART –IRB) at individual organization level offers promise
Can PCORnet e-identify a large number of eligible and approach participants?

<table>
<thead>
<tr>
<th>CDRN</th>
<th>Site</th>
<th>Total Number Approached</th>
</tr>
</thead>
<tbody>
<tr>
<td>MidSouth</td>
<td>Vanderbilt</td>
<td>1664</td>
</tr>
<tr>
<td>REACHnet</td>
<td>Ochsner</td>
<td>2339</td>
</tr>
<tr>
<td>GPC</td>
<td>Iowa</td>
<td>368</td>
</tr>
<tr>
<td>PaTH</td>
<td>UPMC</td>
<td>757</td>
</tr>
<tr>
<td>Capricorn</td>
<td>Northwestern</td>
<td>164</td>
</tr>
<tr>
<td>TOTAL</td>
<td></td>
<td>5292</td>
</tr>
</tbody>
</table>

YES
Can PCORnet e-Engage eligible participants often remotely?

<table>
<thead>
<tr>
<th>CDRN</th>
<th>Site</th>
<th>Total Number Approached</th>
<th>Golden Tickets Entered</th>
<th>% Golden Tickets entered per Approached</th>
</tr>
</thead>
<tbody>
<tr>
<td>MidSouth</td>
<td>Vanderbilt</td>
<td>1664</td>
<td>309</td>
<td>19%</td>
</tr>
<tr>
<td>REACHnet</td>
<td>Ochsner</td>
<td>2339</td>
<td>224</td>
<td>10%</td>
</tr>
<tr>
<td>GPC</td>
<td>Iowa</td>
<td>368</td>
<td>20</td>
<td>5%</td>
</tr>
<tr>
<td>PaTH</td>
<td>UPMC</td>
<td>757</td>
<td>91</td>
<td>12%</td>
</tr>
<tr>
<td>Capricorn</td>
<td>Northwestern</td>
<td>164</td>
<td>23</td>
<td>14%</td>
</tr>
<tr>
<td>TOTAL</td>
<td></td>
<td>5292</td>
<td>668</td>
<td>13%</td>
</tr>
</tbody>
</table>

YES but it varies
Can PCORnet e-randomize patients?

<table>
<thead>
<tr>
<th>CDRN</th>
<th>Site</th>
<th>Total Number Approached</th>
<th>Total Enrolled</th>
<th>% Enrolled Per Approached</th>
<th>% Enrolled Per Golden Ticket Entered</th>
</tr>
</thead>
<tbody>
<tr>
<td>MidSouth</td>
<td>Vanderbilt</td>
<td>1664</td>
<td>144</td>
<td>9%</td>
<td>47%</td>
</tr>
<tr>
<td>REACHnet</td>
<td>Ochsner</td>
<td>2339</td>
<td>82</td>
<td>4%</td>
<td>37%</td>
</tr>
<tr>
<td>GPC</td>
<td>Iowa</td>
<td>368</td>
<td>9</td>
<td>2%</td>
<td>45%</td>
</tr>
<tr>
<td>PaTH</td>
<td>UPMC</td>
<td>757</td>
<td>33</td>
<td>4%</td>
<td>36%</td>
</tr>
<tr>
<td>Capricorn</td>
<td>Northwestern</td>
<td>164</td>
<td>16</td>
<td>10%</td>
<td>70%</td>
</tr>
<tr>
<td>REACHnet</td>
<td>BSW</td>
<td>1</td>
<td>1</td>
<td>100%</td>
<td></td>
</tr>
<tr>
<td>TOTAL</td>
<td></td>
<td>5292</td>
<td>285</td>
<td>5%</td>
<td>43%</td>
</tr>
</tbody>
</table>

YES
Can PCORnet enroll at faster rates than traditional trials?

<table>
<thead>
<tr>
<th>CDRN</th>
<th>Site</th>
<th>Activated</th>
<th>Total Enrolled</th>
<th>Enrollment Rate Pt/site/mth</th>
</tr>
</thead>
<tbody>
<tr>
<td>MidSouth</td>
<td>Vanderbilt</td>
<td>April</td>
<td>144</td>
<td>26</td>
</tr>
<tr>
<td>REACHnet</td>
<td>Ochsner</td>
<td>April</td>
<td>82</td>
<td>15</td>
</tr>
<tr>
<td>GPC</td>
<td>Iowa</td>
<td>Aug</td>
<td>9</td>
<td>4.5</td>
</tr>
<tr>
<td>PaTH</td>
<td>UPMC</td>
<td>Aug</td>
<td>33</td>
<td>16.5</td>
</tr>
<tr>
<td>Capricorn</td>
<td>Northwestern</td>
<td>Sept</td>
<td>16</td>
<td>16</td>
</tr>
<tr>
<td>TOTAL</td>
<td></td>
<td></td>
<td>285</td>
<td>15</td>
</tr>
</tbody>
</table>

YES
Standard CV trial enrollment: 1 pt per month/site
So, could you ever imagine?

- Approaching 700 potential participants in a week
- With a 5 page consent form that was co-created with patients
- That included comprehension testing before randomization
- In a study that collected data from EHR for baseline and follow-up
- With a schedule of assessments and procedures designed with patients
- And enrolled when it was convenient for the participant as opposed to the site….

That is PCORNET.
What’s left?

Can PCORnet Scale?
- Within CDRNs
- Across CDRNs

Can we do it for other studies?

How should we do it?
- Engagement
- Break down the walls
- Leverage what has been built
Russ’ Thoughts on the PCORnet Data Highway

- We have been informatics focused in this talk but much of the innovation to build a viable network is organizational and regulatory.

- Transportation: how many pieces have to come together for you to go travel for the holidays?
  - Walk, Car, Bus, Train, Plane... Boat?
  - Roads: Interstate, state highway, urban street, country road
  - Signs, Licenses
  - Tolls, terminals,
  - Gas, tires:
    - Standard valve stem to pump tire
GPC Structure Hints at the “Research Transportation System”

- Health Systems Advisory Council
- Clinical and Translational Science Advisory Council
- Patient Advisory Council
- Governance Council
  - GPC Leadership
  - Project Management Office
- CDRN Infrastructure
- Data Partners
  - PCORnet Distributed Research Network
  - GPC Infrastructure Core
- Functions
  - Research Opportunity Assessment
  - Sustainability and Pricing
  - Data Request Oversight
  - Administration/Contracting
- Collaborator Engagement
  - Clinicians
  - Investigators
  - Patients
- Functions
  - Standards
  - Software Engineering
  - QA/QC Analytics
  - Interventional Informatics
  - Data Security
  - Study Design
  - Research Question Prioritization
  - IRB/Ethics
  - Biospecimens
  - Recruitment
Historical model of clinical research: Many recruitment sites and a coordinating center

- Hub & spoke model
- Top-down decision-making
- Sites operated independently

Interoperable Networks Share Sites and Data

Each organization can participate in multiple networks

Each network controls its governance and coordination

Networks share infrastructure, data curation, analytics, lessons, security, software development

Other potential partners: disease or treatment-specific networks;
Data Use for Two Masters

How can data help recruit patients to breast cancer trials with greater accuracy and timeliness?

How do we understand integrated data’s ability to provide a complete picture of care for observational studies?

How do we position our sites for NIH/CTSA and PCORnet success?

![Image of a heatmap showing data distribution over time from diagnosis.]
GPC has Pieces to Serve both Customers

Integrating sources at GPC and in support CTSA

- i2b2 alignment for linkage (SHRINE) with other i2b2 CDRNs PaTH/SCILHS
- Ship data to researcher via REDCap

National level intersection

- Shared CDM ETL Harmonization
- PCORnet QC checks and front door
GPC is highly engaged in national studies and seen as a valued contributor

- We’ve implemented IRB Reciprocity & Data Sharing
- Successful proposal to get all the claims for our states from Medicare/Medicaid is seen as leading by our peers
- But our ADAPTABLE accrual is dismal
- The success of our breast cancer survey response and deployment also lets us help develop PCORnet to support Cancer which in turn can help our Cancer Centers
- But we need to retool our obesity efforts
- Most of our data marts in Category 1 and our Category 2 data marts are hopefully ready to join
- But I worry top down nature of current data agreements, infrastructure development, and analysis won’t scale

We are at an inflection point for ourselves and the national network