Our national clinical research system is well-intentioned but flawed

We are not generating the evidence we need to answer the healthcare questions that matter most to patients and their doctors.

- High percentage of decisions are not supported by evidence
- Health outcomes and disparities are not improving
- Current clinical research system faces several problems:
  - Doesn't answer questions that matter most to people
  - Too slow
  - Too expensive
PCORI set out to help us improve

What if we could have at our fingertips trustworthy, high-quality data from health systems, people and partnerships to bring people the real-world answers they seek?

What if we could decrease the time it takes to get clinical insights?

What if we could achieve significant cost savings over a traditional clinical study?
PCORnet®: the National Patient-Centered Clinical Research Network

An innovative initiative funded by the Patient-Centered Outcomes Research Institute (PCORI), PCORnet is a large, highly representative, national patient-centered clinical research network.

Our vision is to support a learning U.S. healthcare system and to enable large-scale clinical research conducted with enhanced quality and efficiency.

Our mission is to enable people to make informed healthcare decisions by efficiently conducting clinical research relevant to their needs.
With PCORnet®, we have developed a nationwide functional research network that...

- **Engages** people, clinicians, and health system leaders throughout
- **Creates** infrastructure, tools, and policies to support rapid, efficient clinical research
- **Utilizes** multiple data sources including electronic health records, insurance claims data, data reported directly by people, and other data sources
- **Enables** people and systems to work collaboratively
Patients, caregivers and others interested in clinical research provide leadership

- **Patients and caregivers** help identify knowledge gaps by sharing their experiences with the daily burden of disease conditions.

- **Patient and stakeholder engagement**, is embodied within the **PCORnet Governance Policy**.

- **Patients and caregivers serve as principal investigators** for PCORnet studies and networks.
PCORnet® embodies a “network of networks” that harnesses the power of partnerships.
PPRNs

ABOUT Patient Powered Research Network (ABOUT Network)
University of South Florida

ARthritis patient Partnership with comparative Effectiveness Researchers (AR-PoWER PPRN)
Global Healthy Living Foundation

CCFA Partners Patient Powered Research Network
Crohn’s and Colitis Foundation

Collaborative Patient-Centered Rare Epilepsy Network (REN)
Epilepsy Foundation

Community and Patient-Partnered Research Network
University of California Los Angeles

Community-Engaged Network for All (CENA)
Genetic Alliance, Inc.

COPD Patient Powered Research Network
COPD Foundation

DuchenneConnect Registry Network
Parent Project Muscular Dystrophy

Health eHeart Alliance
University of California, San Francisco (UCSF)

ImproveCareNow: A Learning Health System for Children with Crohn’s Disease and Ulcerative Colitis
Cincinnati Children’s Hospital Medical Center

Interactive Autism Network
Kennedy Krieger Institute

Mood Patient-Powered Research Network
Massachusetts General Hospital

Multiple Sclerosis Patient-Powered Research Network
Accelerated Cure Project for Multiple Sclerosis

National Alzheimer’s and Dementia Patient and Caregiver-Powered Research Network
Mayo Clinic

NephCure Kidney International
Arbor Research Collaborative for Health

Patients, Advocates and Rheumatology Teams Network for Research and Service (PARTNERS) Consortium
Duke University

Phelan-McDermid Syndrome Data Network
Phelan-McDermid Syndrome Foundation

PI Patient Research Connection: PI-CONNECT
Immune Deficiency Foundation

Population Research in Identity and Disparities for Equality Patient-Powered Research Network (PRIDEnet)
University of California San Francisco

Vasculitis Patient Powered Research Network
University of Pennsylvania
# CDRNs

| **Accelerating Data Value Across a National Community Health Center Network (ADVANCE)** | **National PEDSnet: A Pediatric Learning Health System** |
| Oregon Community Health Information Network (OCHIN) | The Children's Hospital of Philadelphia |

| **Chicago Area Patient Centered Outcomes Research Network (CAPriCORN)** | **New York City Clinical Data Research Network (NYC-CDRN)** |
| The Chicago Community Trust | Weill Medical College of Cornell University |

| **Greater Plains Collaborative (GPC)** | **Patient-Centered Network of Learning Health Systems (LHSNet)** |
| University of Kansas Medical Center | Mayo Clinic |

| **Kaiser Permanente & Strategic Partners Patient Outcomes Research To Advance Learning (PORTAL) Network** | **Patient-oriented SCAlable National Network for Effectiveness Research (pSCANNER)** |
| Kaiser Foundation Research Institute | University of California, San Diego (UCSD) |

| **Research Action for Health Network (REACHnet)** | **PaTH: Towards a Learning Health System** |
| Louisiana Public Health Institute (LPHI) | University of Pittsburgh |

| **Mid-South CDRN** | **Scalable Collaborative Infrastructure for a Learning Healthcare System (SCILHS)** |
| Vanderbilt University | Harvard University |
HPRNs

HealthCore (a subsidiary of Anthem)

Humana – Comprehensive Health Insights (CHI; a subsidiary of Humana Pharmacy Solutions)
Resulting in a national evidence system with unparalleled research readiness

PCORnet represents: ~128 million patients who have had a medical encounter in the past 5 years

*some individuals may have visited more than one Network Partner and would be counted more than once

Pool of patients

For clinical trials: 65,000,000
For observational studies: 128,000,000
A community of research that unites data from patients, clinicians, and systems.
The PCORnet Common Data Model

Data available from several CDRN Network Partners, in the PCORnet CDM and ready for use in research.

Data available at some CDRN Network Partners, may or may not be in the PCORnet CDM and requiring additional work for use in research.
Think of all the different ways you can leverage PCORnet®

PCORnet Front Door: Access point to PCORnet resources

Data
Network Request

Network
Collaborator Request

Study Feasibility Review
PCORnet Study Designation Request
You can use PCORnet® for many kinds of research

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

**Intervventional 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
We've tested our system’s functionality in multiple research settings

14 PCORI-funded PCORnet demonstration studies are answering critical research questions while also testing the infrastructure and key functional aspects of PCORnet.

- Interventional studies
- Observational studies
- Patient-powered research network (PPRN) studies
- Health systems studies

Learn more about [PCORnet Demonstration Studies](#)
We've also amplified our research power with cross-network collaboration

Collaborative research groups:
Catalyzing multi-network research

- Autoimmune and systemic inflammatory syndromes
- Behavioral health
- Cancer
- Cardiovascular health
- Diabetes and obesity
- Health disparities
- Health systems, health policy, and public health
- Hospital medicine
- Kidney health
- Pediatrics
- Pulmonary
Here's how the PCORnet® distributed research network works

The Requestor 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 Requestor
The Front Door is open…
take your research to the next level

Through PCORnet Front Door, we invite PCORnet researchers and other investigators, patient groups, healthcare organizations, clinicians or clinician groups, government and industry scientists, and sponsors to collaborate on important patient-centered clinical research studies.

Check it Out!
http://pcornet.org/frontdoor/
Even more enhancements in data are coming soon

- Claims data
- People-reported data
- Medicare data
- Registry data
More information on PCORnet®

- Website: www.pcornet.org
- PCORnet Commons: http://pcornetcommons.org/
- Twitter: @PCORnetwork
- YouTube: PCORI YouTube Playlist
- Vimeo: PCORI Vimeo Playlist
- PCORnet communications contact: Jennifer.Cook@duke.edu
PCORnet® and PCORI

About PCORnet
PCORnet, the National Patient-Centered Clinical Research Network, is an innovative initiative of the Patient-Centered Outcomes Research Institute (PCORI). The goal of PCORnet is to improve the nation's capacity to conduct clinical research by creating a large, highly representative network that directly involves patients in the development and execution of research. More information is available at www.pcornet.org.

About PCORI
The Patient-Centered Outcomes Research Institute (PCORI) is an independent, nonprofit organization authorized by Congress in 2010. Its mission is to fund research that will provide patients, their caregivers, and clinicians with the evidence-based information needed to make better-informed healthcare decisions. PCORI is committed to continually seeking input from a broad range of stakeholders to guide its work. More information is available at www.pcori.org.
Appendix

Additional slides about the CDM
Additional slides about research & case studies
Additional slides about engagement
Additional slides about the CDM
Underpinned by a Common Data Model
Same data are represented differently at different institutions (e.g., Type of Encounter)

In order to be able to trust results of an analysis, we need to have consistent representations
Underpinned by a Common Data Model
Same data are represented differently at different institutions (e.g., Race)

In order to be able to trust results of an analysis, we need to have consistent representations
PCORnet® Common Data Model domains

- Based on FDA Sentinel Common Data Model
- Licensed under Creative Commons (open-access, use, and share)
- Designed to promote multi-site, patient-centered research
- Allows for interoperability

PCORnet Common Data Model Domains, v3.0 and v3.1

**DEMOGRAPHIC**
Demographics record the direct attributes of individual patients.

**ENROLLMENT**
Enrollment is a concept that defines a period of time during which a person is expected to have complete data capture. This concept is often insurance-based, but other methods of defining enrollment are possible.

**ENCOUNTER**
Encounters are interactions between patients and providers within the context of healthcare delivery.

**DIAGNOSIS**
Diagnosis codes indicate the results of diagnostic processes and medical coding within healthcare delivery. Data in this table are expected to be from healthcare-mediated processes and reimbursement drivers.

**PROCEDURES**
Procedure codes indicate the discreet medical interventions and diagnostic testing, such as surgical procedures and lab orders, delivered within a healthcare context.

**VITAL**
Vital signs (such as height, weight, and blood pressure) directly measure an individual’s current state of attributes.

**LAB_RESULT_CM**
Laboratory result Common Measures (CM) use specific types of quantitative and qualitative measurements from blood and other body specimens. The common measures are defined in the same way across all PCORnet networks, but this table can also include other types of lab results.

**CONDITION**
A condition represents a patient’s diagnosed and self-reported health conditions and diseases. The patient’s medical history and current state may both be represented.

**PRO_CM**
Patient-Reported Outcome (PRO) Common Measures (CM) are standardized measures that are defined in the same way across all PCORnet networks. Each measure is recorded at the individual item level: an individual question/statement, paired with its standardized response options.

**DISPENSING**
Outpatient pharmacy dispensing, such as prescriptions filled through a neighborhood pharmacy with a claim paid by an insurer. Outpatient dispensing may not be directly captured within healthcare systems.

**PREScribing**
Provider orders for medication dispensing and/or administration. These orders may take place in any setting, including the inpatient or outpatient basis.

**PCORNET_TRIAL**
Patients who are enrolled in PCORnet clinical trials.

**DEATH**
Reported mortality information for patients.

**DEATH_CAUSE**
The individual causes associated with a reported death.

**HARVEST**
Attributes associated with the specific PCORnet datamart implementation, including data refreshes.
Additional slides about research
PCORnet® research

Types
- Comparative effectiveness
- Population health
- Health care delivery
- Patient-centered research

Data sources
- Electronic health records
- Patient-reported registries
- Patient-reported outcomes
- Clinical registries
- Health plan claims

Study designs
- Randomized controlled trials
- Cluster randomized trials
- Nonrandomized comparative trials
- Longitudinal studies
- Surveillance
- Cross-sectional
With coverage in every state — PCORnet® represents thousands of conditions

This map depicts the number of PCORI-funded Patient-Powered or Clinical Data Research Networks that have coverage in each state.
A few case examples

Observational studies
- Obesity studies
  - PCORnet Bariatric Study
  - Short- and Long-term Effects of Antibiotics on Childhood Growth

Pragmatic clinical trials
- ADAPTABLE
- INVESTED
Let's take a look at what PCORnet® can do via a demonstration study

The ADAPTABLE Aspirin Study

THE QUESTION

Clinicians often prescribe aspirin to prevent strokes and heart attacks in people living with heart disease. Research has yet to determine the best dose to use, since aspirin can cause serious side effects – like bleeding – in some people.

THE STUDY

The ADAPTABLE trial will compare two common aspirin dosages.

- 325 mg
- 81 mg

The study will be large and will involve patients across the U.S.

- 20,000 patients living with heart disease will use a daily aspirin dose of either 81 mg or 325 mg.

ADAPTABLE will use PCORnet to conduct the study and disseminate results. Patients will be partners at every stage of the trial, which will collect data using tools with state-of-the-art security.
What if a choice made over the counter prevented...

19,000 deaths/heart attacks or thousands of bleeds annually in the United States
INVESTED

Post-MI or HF hospitalization

N = 9300

Duration
3 influenza seasons + vanguard season

RANDOMIZED 1:1 DOUBLE-BLIND ANNUAL VACCINE STRATEGY

High-dose trivalent influenza vaccine

All other CV Rx per treating MD

Followed up to 4 times a year ANNUAL VACCINATION to assigned strategy

Standard-dose quadrivalent influenza vaccine

Primary endpoint:
Death or cardiopulmonary hospitalization

*with 1 additional CV risk factor

Age ≥ 65
LVEF < 40%
DM
BMI > 30
eGFR < 60
Hx ischemic stroke
Hx PAD
Current smoking

Duration
3 influenza seasons + vanguard season

pcornet®
PCORnet obesity studies

**Bariatric study**
- **Aim:** to provide accurate estimates of 1-, 3-, and 5-year benefits and risks of three main surgical treatment options for severe obesity
  - Roux-en-y gastric bypass (RYGB)
  - Adjustable gastric banding (AGB)
  - Sleeve gastrectomy (SG)

**Childhood obesity study**
- **Aim:** compare effects of different types, timing, and amount of antibiotics used in the first two years of life with BMI and obesity at ages 5 and 10
  - And explore how different factors such as gender, race/ethnicity, geographical location, or characteristics of the mother such as her BMI or type of delivery may impact outcomes
### PCORnet obesity studies

<table>
<thead>
<tr>
<th>Bariatric</th>
<th>Antibiotics</th>
</tr>
</thead>
<tbody>
<tr>
<td># Participating CDRNs</td>
<td>11</td>
</tr>
<tr>
<td># Participating institutions</td>
<td>55</td>
</tr>
<tr>
<td># Patients</td>
<td>65,088 (64,184 adults; 904 adolescents)</td>
</tr>
</tbody>
</table>
Additional slides about engagement
PCORnet® engagement assessment tool

The engagement assessment tool to ensure people remain central to our work.

The tool is available to the public on the PCORnet Commons.
Engagement in action: Antibiotics Study

The PCORnet Antibiotics Study formed an Executive Antibiotics Stakeholder Advisory Group (EASAG) to work alongside the scientific team to carry out the study’s objectives.

Members include parents, caregivers, pediatricians, pharmacists, investigators, and advocacy/health systems leaders.
Use the PCORnet® Commons!

The PCORnet Commons is a place for people striving to realize the vision of people-centered research.

The goal of the PCORnet Commons is to provide opportunities to increase collaboration, efficiency, and people-centeredness in clinical research.

http://pcornetcommons.org/
The PCORnet® Commons allows you to:

- Connect with real people in PCORnet and beyond
- Discover tools, templates, and more
- Read about innovative case studies and experiences
- Start a group to discuss what's important to your community in real-time
- Explore today’s most promising practices in clinical research