

# Building a National Clinical Research Infrastructure

**OneFlorida  
3<sup>rd</sup> Annual Stakeholder Meeting  
University of Florida**

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Executive Director, PCORI  
November 28, 2016**



# Evolving Vision of a Research Infrastructure

History of PCORnet – from the beginning!

PCORnet Today

The Future – Changing the Research Culture



# Our national clinical research system is well-intentioned but flawed!

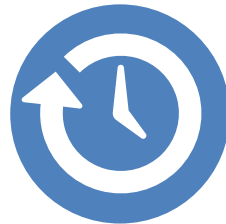
- High percentage of decisions are not supported by evidence

We are not generating the evidence we need to support the healthcare decisions that patients and their doctors have to make every day.

- Current clinical research system faces several problems:



Doesn't answer questions that matter most to people



Too slow



Too expensive



# PCORnet's Mission

- **Faster, easier, and less costly** clinical and health system research, **both observational studies and randomized trials**
- Harness the power of large amounts of electronic health record data and patient partnerships, and by **making contracting negotiations and IRB approval/oversight more efficient.**
- To embed the research within health systems and use data and research findings to facilitate **health system improvement.**
- And in the process, **transform the culture of clinical research** from one directed by researchers acting as entrepreneurs to one driven by collaboration, data sharing, and the needs of patients, clinicians, systems and payers.



# The Vision of a National Research Infrastructure



## ONC

- Meaningful Use
- Nationwide Health Information Network
- Federal Health Architecture
- EHR Certification Standards & Interoperability Framework



## FDA

- Sentinel Initiative
- OMOP
- Reagan-Udall
- NEST



## AHRQ

- DRNs
- PBRNs
- **Registries**
- SPAN
- PROSPECT
- EDM Forum



## NIH

- SEER Registries
- CTSA
- Collaboratory
- CRN, CVRN
- ClinicalTrials.gov
- eMERGE Network
- PROMIS/ NIH - Snomed-CT, LOINC



## VA

- VistA
- iEHR (2017)



## IOM

2011 Report: *Digital Infrastructure for the Learning Health System: The Foundation for Continuous Improvement in Health and Health Care*

## Payers



## Specialty Societies



## Industry



Mainspring Data Express

## Innovators and Entrepreneurs



patientslikeme

CANCERCOMMONS





# A PCORI Workshop to Advance the Use of Electronic Data in Conducting PCOR at Stanford University



***Bringing together more than 70 national leaders with expertise in:***

- Outcomes Research
- Informatics
- Methods
- Privacy and Ethical Issues
- Patient-centeredness

***Featuring Exploration of Research Models including:***

- C3N
- DARTNet
- HMORN
- SCANNER
- Sentinel and mini-Sentinel
- Registry of Registries

***Soliciting action items in the following areas:***

- Governance
- Data standards and interoperability
- System architecture and data exchange models
- Privacy and ethical issues,
- Methods
- Electronic capture of patient reported outcomes (PROs)
- Unconventional approaches, for example the opportunities to leverage activated patient networks for research



**Live-streaming at [pcori.org](http://pcori.org) July 2-3, 2012**

# Two Distinct (and Complementary) Approaches Emerge from Palo Alto



PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

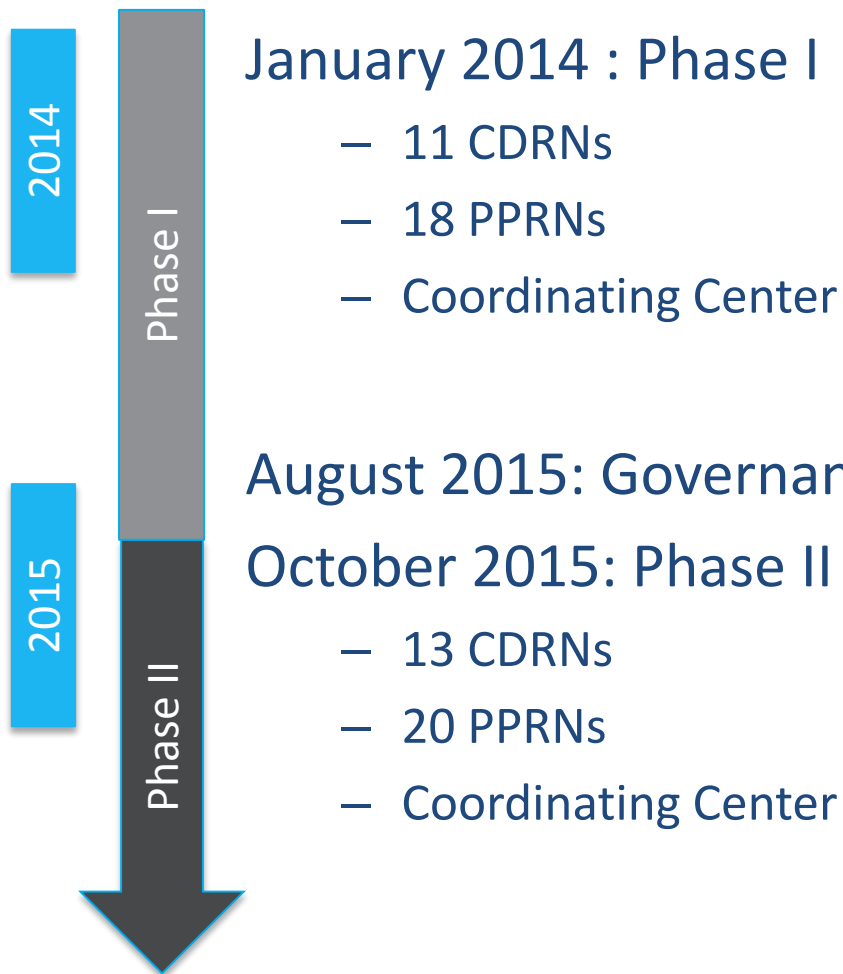
## PCORI National Workshop to Advance Use of Electronic Data



Clinical Data  
Network

Patient-Powered  
Research  
Network

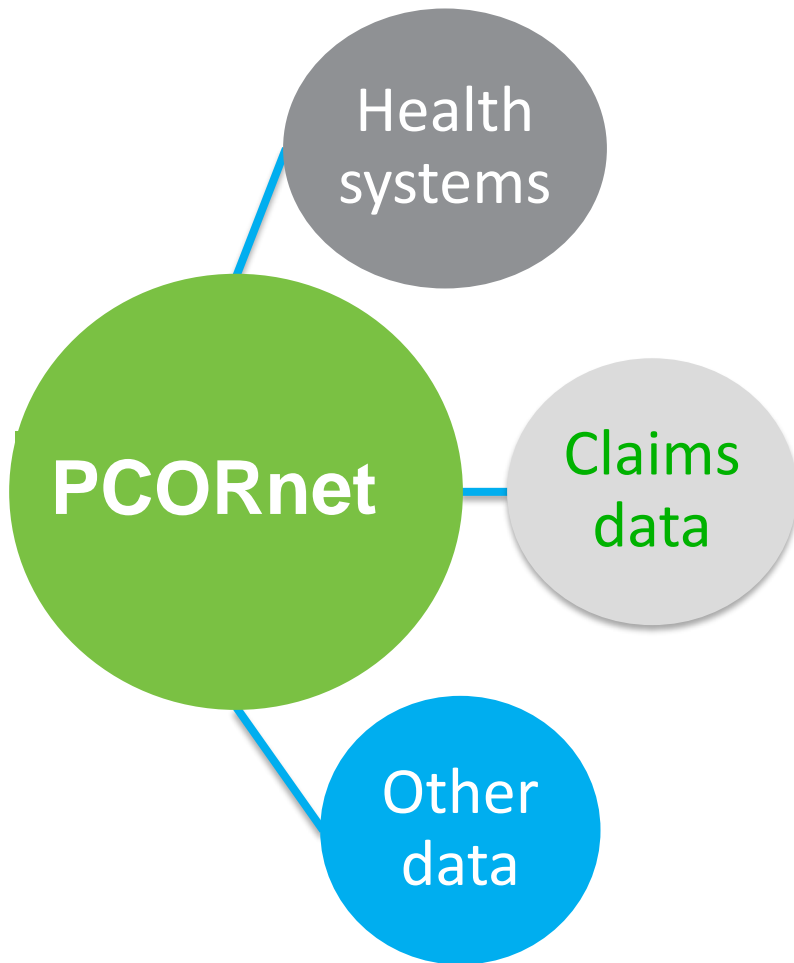
# PCORnet Investment Timeline 2014-2016



*In July 2012, PCORI set out to fill in a large gap in the US national research infrastructure that was slow, costly, and often produced results that were not meaningful to patients.*



# PCORnet Clinical Data Research Networks (CDRNs) – Phase II



- The Chicago Community Trust (CAPriCORN)
- The Children’s Hospital of Philadelphia (PEDIStnet)
- 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)

# PCORnet Patient-Powered Research Networks (PPRNs) – Phase II

- University of South Florida (ABOUT Breast Cancer Network)
- Global Health Living Foundation (AR-PoWER)
- Mayo Clinic (Alzheimer's 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 (IANv- autism)
- 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)

# PCORnet Achievements 2014-2016

From vision of a strong involvement of participants in all phases of research and use electronic health data in conducting comparative effectiveness research....

...to implementation in two Phases:

## Phase I focused on:

- building the network, with focus on governance;
- developing individual CDRNs and PPRNs;
- laying out a common data infrastructure for distributed research.

## Phase II focused on completing remaining building blocks:

- developing sustainability plans;
- administrative simplicity;
- implementing the Distributed Research Network for observational studies and pragmatic randomized trials;
- launching demonstration projects.

# PCORnet



**130** health systems across  
the country

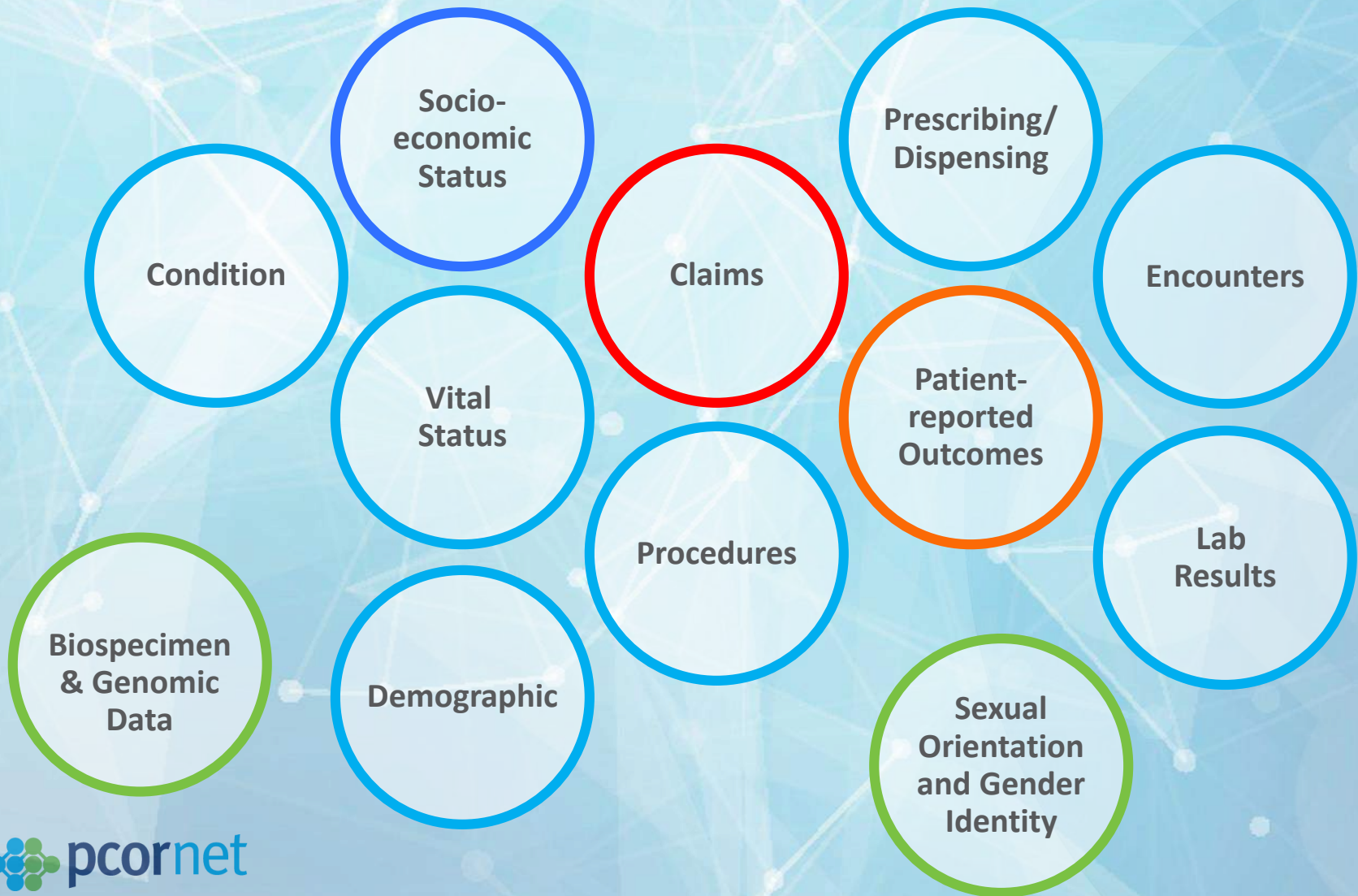
**Over 80 data marts**

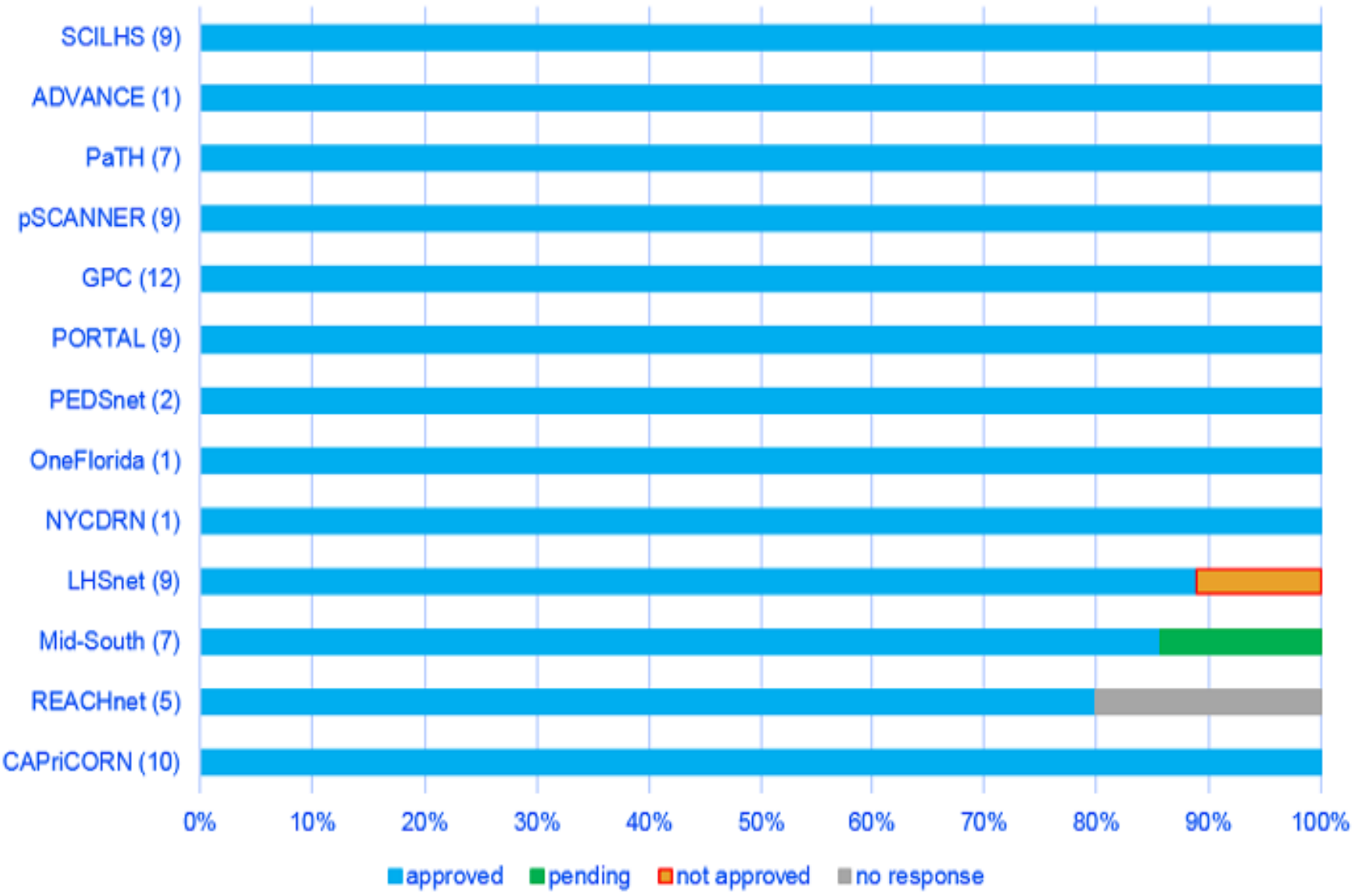
Data on

**>100 million patients**

# Supported by a Common Data Model

-  Data domains in the CDM
-  Domains that can be added





## PCORnet Status Update for CDRNs

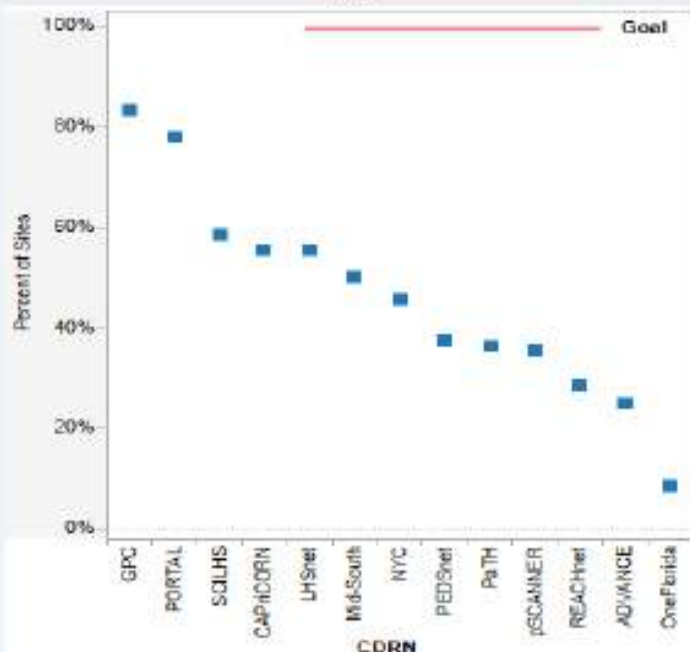
Updated 1/3/2017

### RATIONALE 1 SMART IRB

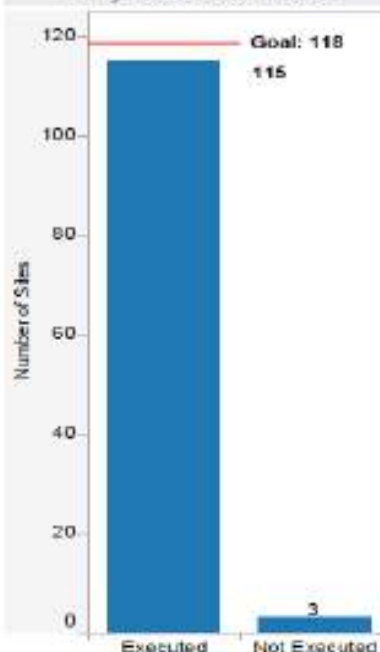
### RATIONALE 2 Data Sharing Agreement (DSA) V.1.0

### RATIONALE 3 Query Fulfillment

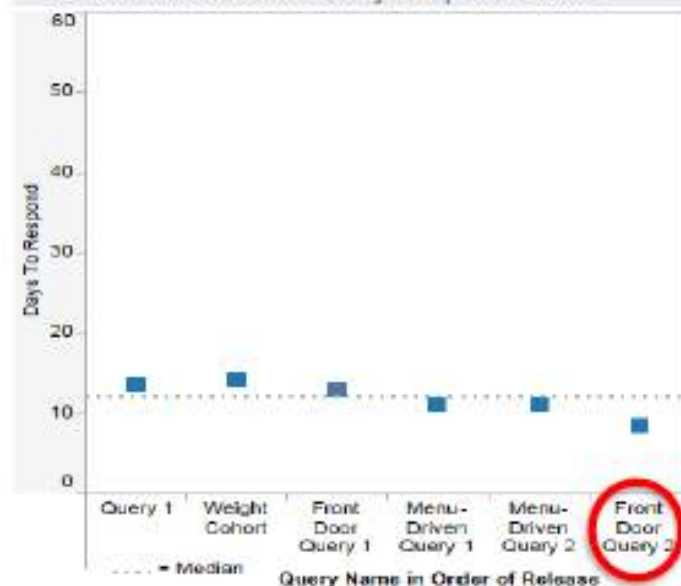
Percent of CDRN sites who have executed SMART IRB



Number of Sites with Fully-Executed DSAs



Median Network Query Response Time\*



\*Network Partners that did not respond to the queries are excluded from the average.

**RATIONALE 1:** NIH will require a single IRB for sites participating in federally-funded multi-site trials beginning in September 25, 2017. PCORnet is partnering with NCATS to support sites to sign on to the SMART IRB single IRB model and obtain a fully executed joinder agreement.

**RATIONALE 2:** The Data Sharing Agreement (DSA) defines the standard terms to which the PCORnet Coordinating Center (Harvard Pilgrim and Duke) will adhere when information/data are sent from the Network Partners to CDRN the Coordinating Center. A total of 97.5% of eligible CDRNs and PPRNs (sites) have signed an initial "V.1.0" Data Sharing Agreement. Version 2.0 (V.2.0) of the DSA is anticipated for release in March of 2017 and will be made available to the Network Partners to sign.

**RATIONALE 3:** Query Fulfillment is a complex, iterative process requiring knowledge of PCORnet Network Partner data, the common data model, existing PCORnet SAS programs or tools (e.g., PMP1, MDQ) or requests for SAS program development. SAS programmed queries and menu-driven queries (MDQ) have been released to the Network Partners for query fulfillment. Quickly responding to these query requests by providing output data that contributes to a final report is essential to the success of PCORnet. Network Partners only receive queries that are relevant to them to respond to (e.g., because of the population or topic of the query).

# Spotlight on Demographics Data

2014 PCORnet Population Characteristics	2014 PCORnet Population
	N
<b>Overall (N)</b>	41,216,568
<b>Age*</b>	
0-20	11,361,889
21-44	11,589,633
45-64	10,951,968
65-74	4,156,901
75+	3,156,017
<b>Sex*</b>	
Female	23,537,224
Male	17,667,683
Other/Missing	11,661
<b>Race*</b>	
Black/African American	4,979,389
White	25,669,541
Other/Missing	10,537,665
Hispanic	
Yes	5,870,400
No	23,357,500
Other/Missing	9,988,694

Based on individuals who received care during 2014 at any of the 50 Network Partner institutions that contributed data available as of August 1, 2015.



\*Some stratified counts may not equal the total N counts because of low cell count masking.



# Overall Counts

<b>Condition</b>	<b>PCORnet</b>
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Respiratory conditions	2,837,803
Selected malignancies	1,294,158
Myocardial infarction	354,929
Stroke	420,802
Rheumatoid arthritis	254,803
Ulcerative colitis	88,029
Hypertension	5,902,641
Renal disease	1,018,729
Influenza/pneumonia	869,306



# PCORnet Research Demonstration Projects: Funded by PCORI

Study	Study Design	Areas of focus	Network Goals
ADAPTABLE (1)	RCT	<ul style="list-style-type: none"> <li>- Comparing two doses of Aspirin in persons with pre-existing CVD</li> </ul>	To pilot approaches to e-identification, e-consent and e-follow up
Obesity Studies (2)	Observational	<ul style="list-style-type: none"> <li>- Comparing outcomes for 3 common bariatric surgery techniques</li> <li>- Effect of antibiotic use in children on weight gain</li> </ul>	To develop DRN and governance to run rapid queries in large scale network
PPRN Projects (6)	RCT, Observational, N-of-1	<ul style="list-style-type: none"> <li>- Depression</li> <li>- Autoimmune and inflammatory diseases</li> <li>- Cardiovascular disease</li> <li>- Chronic Obstructive Pulmonary Disease (COPD)</li> <li>- Obstructive Sleep Apnea</li> </ul>	To answer research questions driven by patients, using PCORnet resources
Cross-PPRN (1)	RCT	<ul style="list-style-type: none"> <li>- 100+ chronic conditions in PPRNs</li> </ul>	To engage all PPRN communities and test rapid recruitment
Health Systems (4)	Observational	<ul style="list-style-type: none"> <li>- High utilization</li> <li>- Automating Quality and Safety Benchmarking for Children</li> <li>- Case Management</li> </ul>	To provide rapid answers to health systems of research institutions

# ADAPTABLE: Enabling Pragmatic Research: eScreening, eEnrollment and eFollowup



**ADAPTABLE**  
Enrollee



*Baseline Data*



OR



**Adaptable**

The Aspirin Study

## Call FOLLOW-UP

- *Patient Reported Outcomes*
- *Medication use*
- *Health outcomes*

## Portal FOLLOW-UP

- *Patient Reported Outcomes*
- *Medication use*
- *Health outcomes*



## PCORNet Coordinating Center FOLLOW-UP

- *Via Common Data Model*
- *Longitudinal health outcomes*



## CMS & Payer Virtual Data Warehouse FOLLOW-UP

- *Longitudinal health outcomes*

<http://adaptablepatient.com>

# Traditional Trials vs. ADAPTABLE

	<b>Traditional</b>	<b>ADAPTABLE</b>
Inclusion/Exclusion criteria review	Sample via monitor visit	EHR and CDM (Common Data Model)
Representative cohort	Narrow	Broad
Consent	In Person Facilitated	Patient-directed
Comprehension tested	No	Yes
Format	Paper	Electronic (e-consent)
Data collection	Patient-reported Site-recorded	Patient-reported CDM
Source documents	Only seen by site	EHR data via CDM
Endpoint adjudication	Yes	No
Patient involvement	As participants only	Protocol design, DSMB, analyses, dissemination, as well as participants








# Collaborative Research Groups (CRGs) in PCORnet

- Brings research, clinical and stakeholder expertise collaborating in specific areas of interest that align with federal research priorities (NIH, CDC, FDA, AHRQ) and PCORnet's strengths
- Primary goal is to catalyze new multi-Network research by:
  - Advancing the science of patient-centered outcomes and pragmatic research
  - Creating research teams from multiple networks to develop proposals for high priority research questions
  - Developing common data elements pertinent to clinical area
  - Engaging stakeholders and funders
  - Assisting the PCORnet Front Door

Examples of potential CRGs include:

Cancer  
Pediatrics  
Cardiovascular  
Health Disparities  
Behavioral Health  
Autoimmune  
Health Care Delivery

# Approved CRGs & Lead PIs

-  Autoimmune and Systemic Inflammatory Syndromes  
*Ben Nowell, Peter Merkel*
-  Behavioral Health  
*Scott Stroup, Sheryl Kataoka*
-  Cancer  
*Elizabeth Chrischilles, Sue Friedman, Debra Ritzwoller*
-  Cardiovascular Health  
*Mark Pletcher, Veronique Roger, Rhonda Cooper-DeHoff*
-  Diabetes and Obesity  
*John Buse, Russell Rothman, Desmond Schatz*
-  Health Disparities  
*Lewis Raynor, Mitchell Lunn*
-  Health Systems, Health Policy and

## Public Health

*Rainu Kaushal, Elizabeth Shenkman*

## Hospital Medicine

*Andrew Auerbach, Sunil Kripalani, David Meltzer*

## Kidney Health

*Laura Mariani, Debbie Gipson, Michael Matheny, Edward Siew*

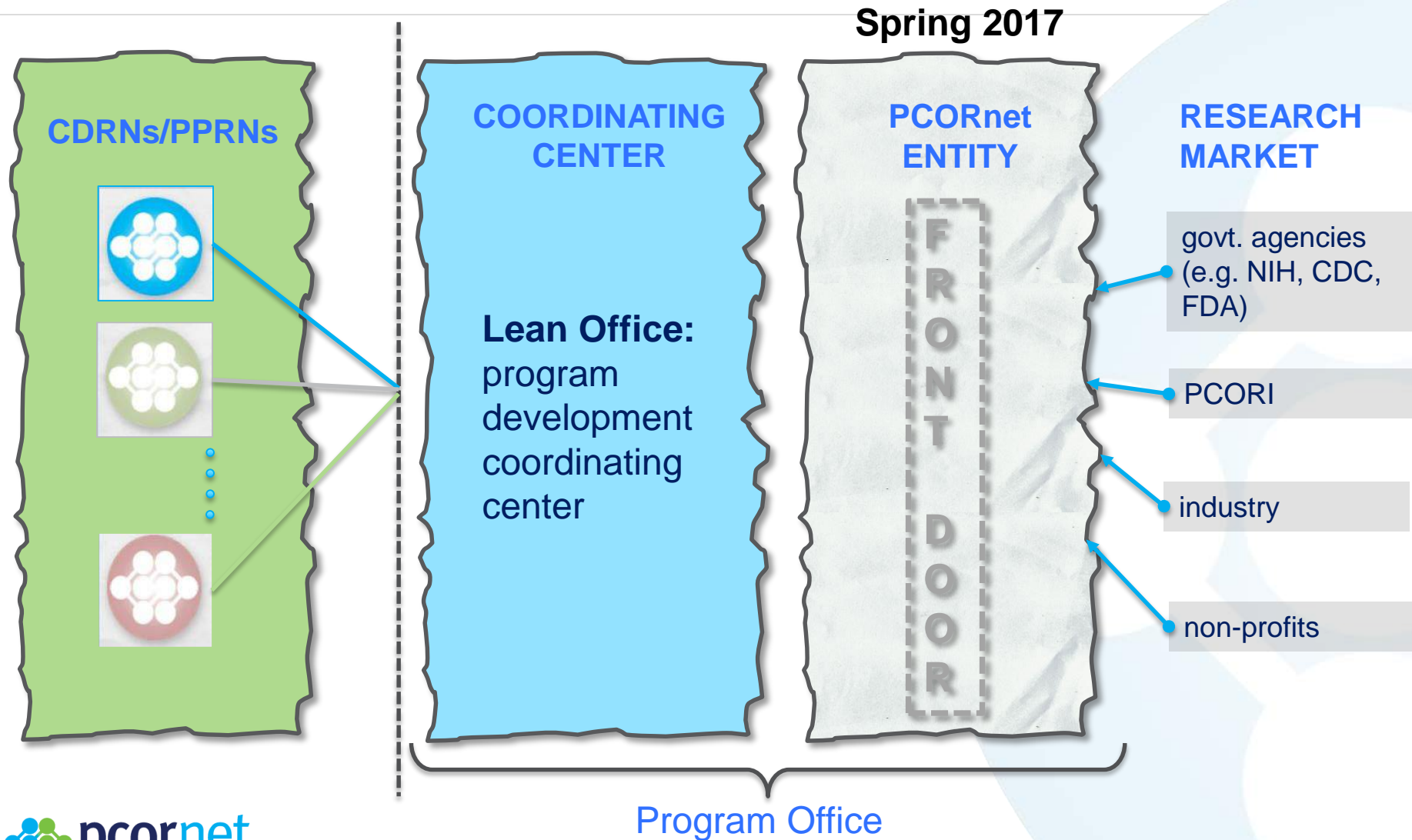
## Pediatrics

*Chris Forrest, Elizabeth Shenkman*

## Pulmonary

*Rebecca Bascom, Dave Mannino*

# PCORnet is Going Independent!



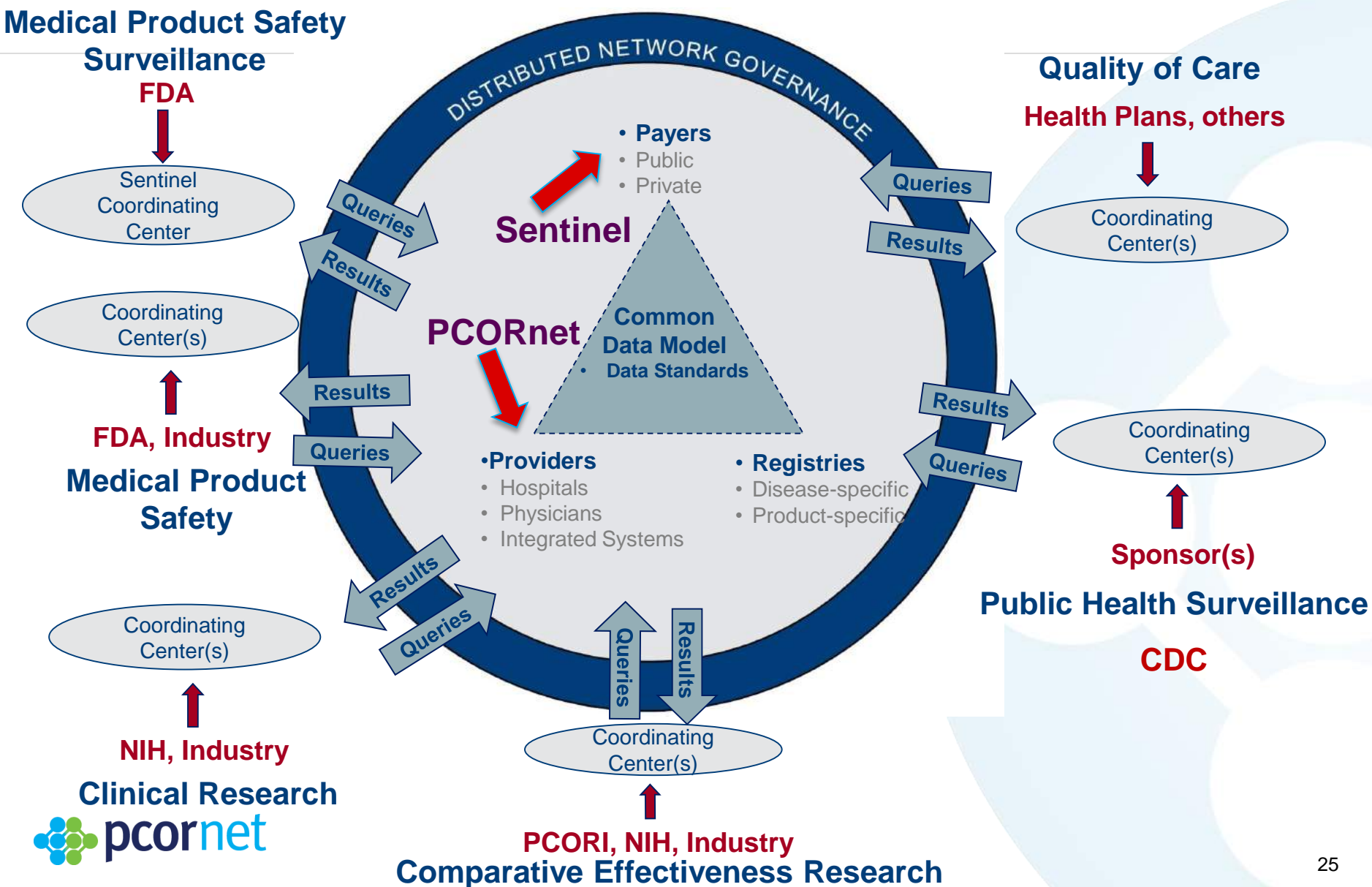
# PCORnet Front Door



- 🌐 October 2016 – Internal Network Launch for data and collaboration requests
- 🌐 Spring 2017 – National Public Launch
- 🌐 Intended for PCORnet researchers and other investigators, patient groups, healthcare organizations, clinicians, clinician groups, government, industry scientists, and sponsors for the conduct of more efficient, trustworthy clinical research.



# PCORnet as Part of a National Evidence Generation Infrastructure



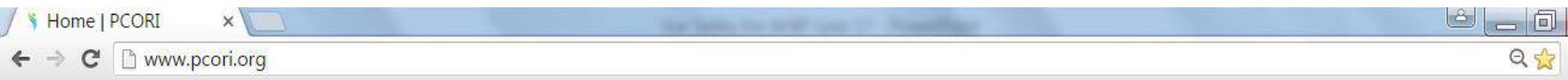
# So, what are PCORI and PCORnet Trying to Change?

- ❖ **Who's on the Research Team** – adding patients, and other stakeholders, especially clinicians
- ❖ **The Kinds of Questions Asked** – questions that matter to patients, caregivers, clinicians, delivery systems and payers; questions whose answers are likely to change practice
- ❖ **How Data are Analyzed** – toward personalized approach
- ❖ **The Speed of Implementation** – from 17 years to ???
- ❖ **The Role of the Researcher** – from solo entrepreneur to team member.

Thoughts?



# Thanks..... and Stay in Touch!



Patient-Centered Outcomes Research Institute

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## Caregivers Take Center Stage

Family care providers play a major role in the lives of people with dementia—and in a PCORI-funded PCORnet partner network.

[FIND OUT MORE >](#)

