

PCORnet: A Platform to Transform Clinical Research

January 2018



pcornet[®]

The National Patient-Centered
Clinical Research Network



 Problem with current system

 What pcorNet is

 What pcorNet is becoming

Our national clinical research system is broken

We are not generating the evidence we need to answer the health 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
- ❁ Medicines and medical care are too expensive
- ❁ Current clinical research system is not working well

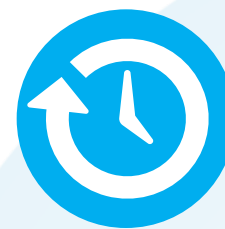
Our national clinical research system is broken



**Too many fail
w/o data**



**Doesn't answer
questions that
matter most to
people**



**Too
slow**



**Results not
adopted**



**Too
expensive**



**Data Not
Shared**

Why clinical trials fail

- ⚙️ Safety – expected, partly due to inadequate models
- ⚙️ Efficacy – expected, partly due to inadequate model
- ⚙️ Design – trial design does not take into account key facts, variables
- ⚙️ Power – a key design problem. Too many under powered studies
- ⚙️ Funding/staff – run out of money or key staff depart
- ⚙️ Failure to accrue – unable to get enough research volunteers

Sad data

- 🌐 About 5% of all trials terminate without producing data.
- 🌐 Biggest single cause is low or insufficient accrual (35-40%).
- 🌐 Failed trials due to failed accrual means
 - No data
 - Lost investment
 - Failure to be true to promise to participants
- 🌐 Very little data on recruitment methods. Most is hypothetical
- 🌐 What would be the economic, ethical, scientific, and health impacts of increasing accrual rates?

Participant engagement: Dire need for evidence

- Most “data” about participant engagement in research is hypothetical.
- Empirical evidence is from small, underpowered studies, usually in specific populations with specific interventions.
- Lot's of attention on patient/participant engagement. Need action
- Cochrane study shows insufficient evidence to show that limited tested methods work.

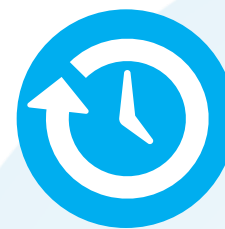
Our national clinical research system is broken



**Too many fail
w/o data**



**Doesn't answer
questions that
matter most to
people**



**Too
slow**



**Results not
adopted**




**Too
expensive**



**Data Not
Shared**

PCORI set out to help improve research enterprise



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[®] is a “network of networks”



20 Patient-Powered Research Networks + 13 Clinical Data Research Networks + 2 Health Plan Research Networks + 1 Coordinating Center = A national infrastructure for people-centered clinical research

national research system with unparalleled scale and capacity

139 healthcare organizations; **104** participate in data network

128 million people's data in the data network; **65 million** eligible for clinical trials

All health conditions (**100,000+** ICD10 codes; **300,000+** SNOMED codes)

All healthcare specialties and sub-specialties

All service settings

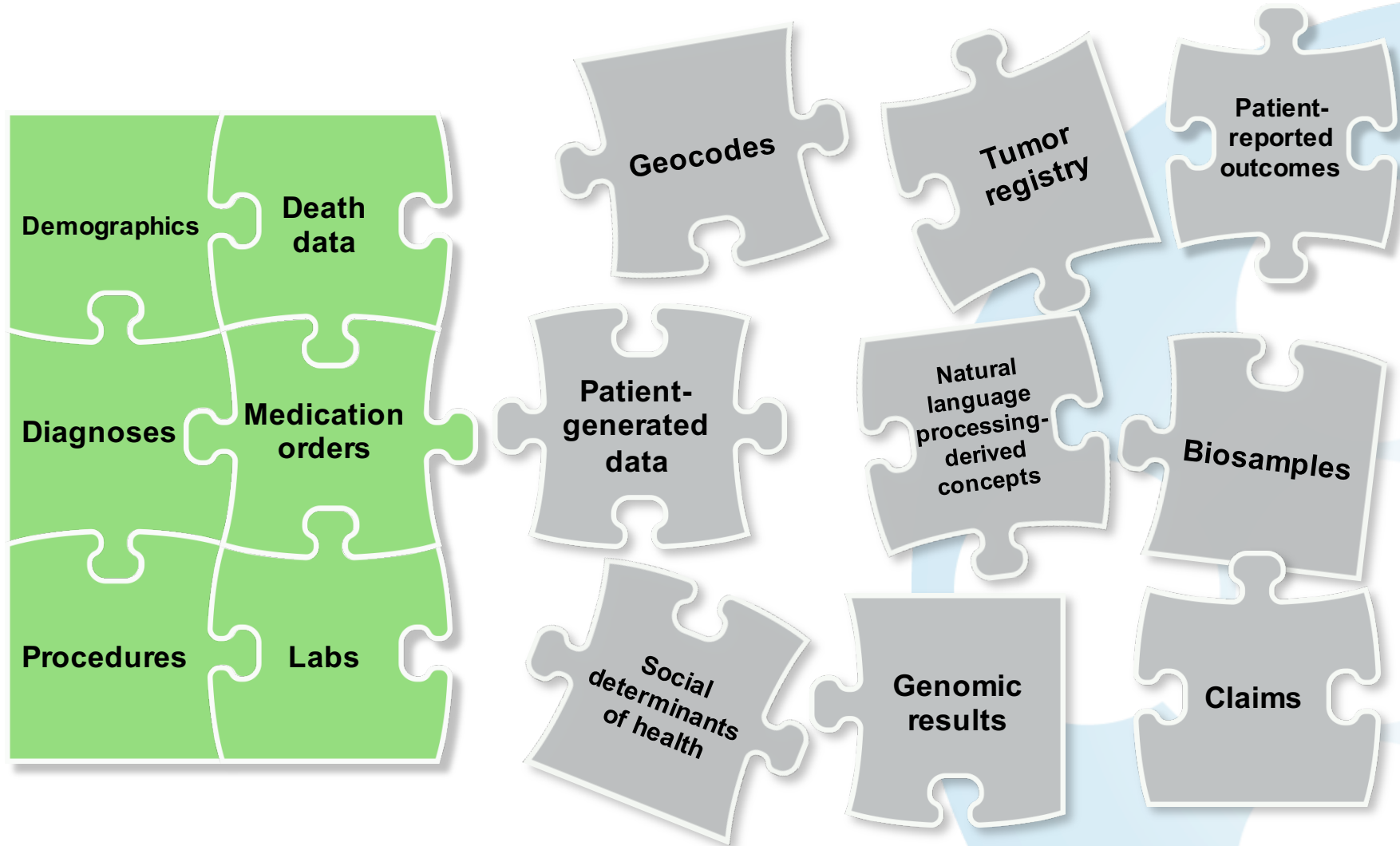
What makes PCORnet special?

 Data

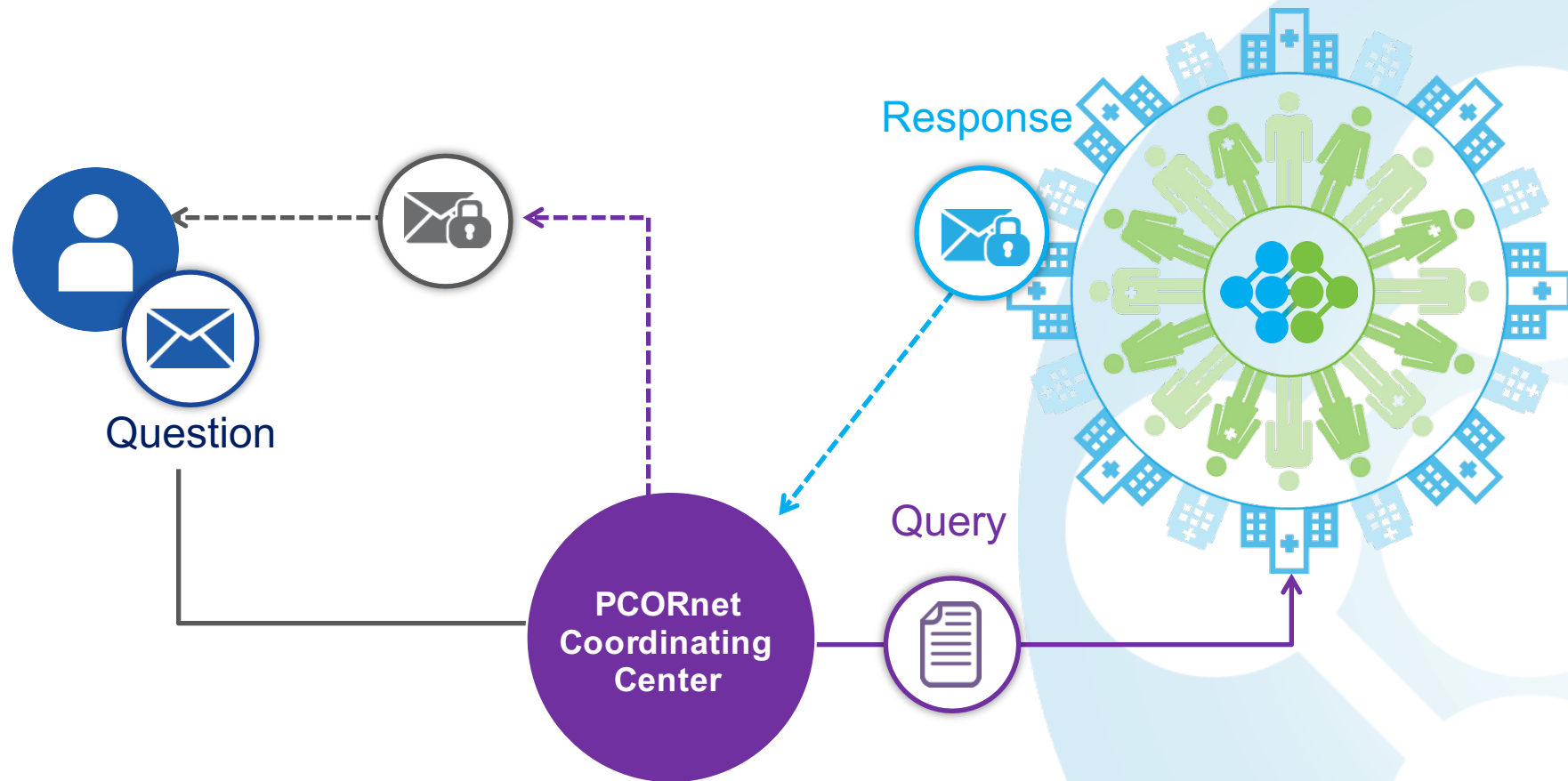
 Trial infrastructure and readiness

 People-centeredness

The PCORnet Common Data Model



Data queries in the PCORnet[®] distributed research network



Example of Data Network Request

- prep-to-research queries to assess the number of patients aged 65 years and older with:
 - a diagnosis of Parkinson's in a set time period **(58,408 identified)**
 - a diagnosis of Parkinson's in a set time period AND a diagnosis of any fracture within a year following the index date **(5,193 identified)**
 - a diagnosis of Parkinson's in a set time period AND a diagnosis of a hip fracture within a year following the index date **(1,205 identified)** Results were provided in aggregate and by Network Partner
 - 50 network partners responded

Testing PCORnet functionality

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
- ❖ Health systems studies

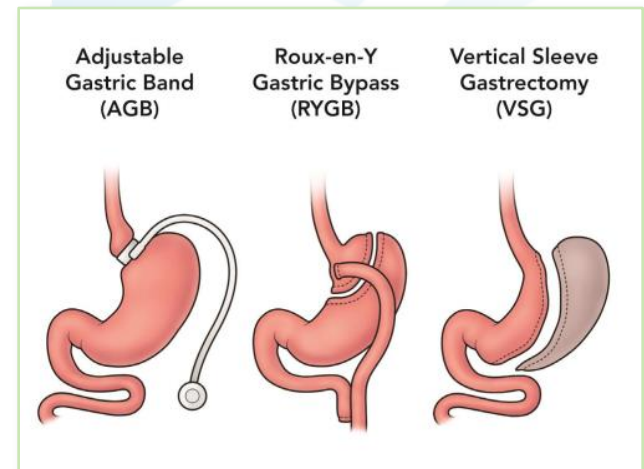
Interventional Clinical Trials

- ❁ **ADAPTABLE:** optimal dose of aspirin (325 vs 81mg) enrolling >500/month
- ❁ **COMBINE:** biologics +/- methotrexate in pediatric Crohn's disease
- ❁ **RELIANCE:** Roflumilast or Azithromycin to prevent COPD Exacerbations
- ❁ **INVESTED:** INfluenza Vaccine to Effectively Stop Cardio-Thoracic Events and Decompensated Heart Failure
- ❁ **Healthy Mind Healthy You:** online mindfulness-based treatment ("standard", 8 sessions v "light", 3 sessions)

Observational Study: Bariatric

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

# Participating Clinical Data Research Networks	11
# Participating institutions	55
# Patients	65,088 (64,184 adults; 904 adolescents)



Sustainability



PCORI was authorized and funded in the ACA through FY2019.



PCORnet was envisioned to be self-sustaining beyond PCORI funding



PCRF was created to lead PCORnet to the next stage

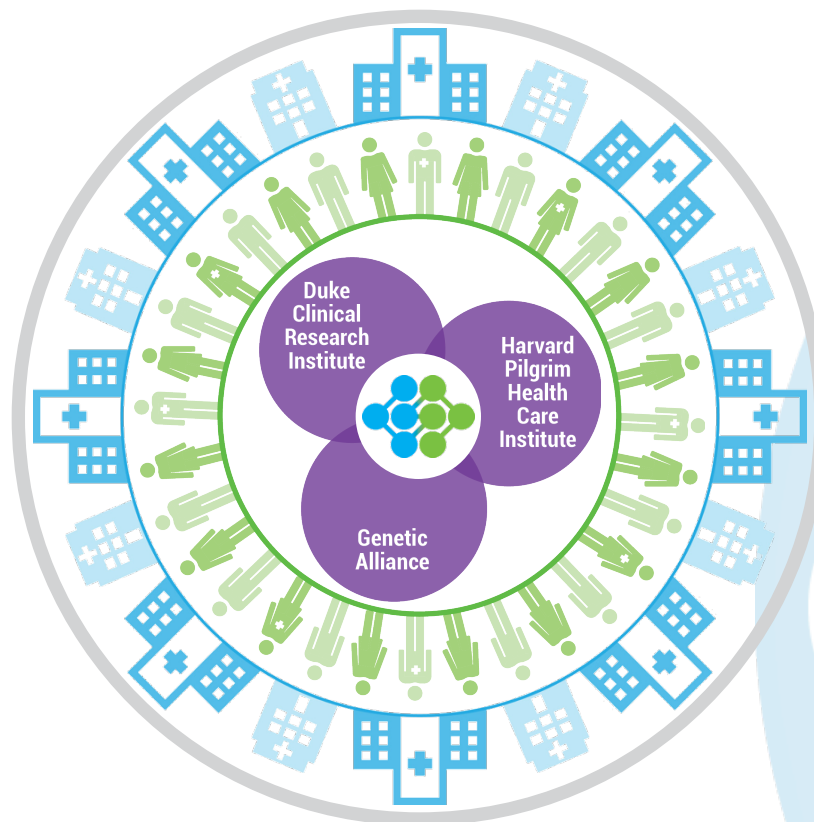


PCRF's inaugural board


People-Centered
RESEARCH FOUNDATION

- **CHAIR: Robert Califf**, former FDA Commissioner, now at Duke University and Verily
- **Richard Bankowitz**, executive vice president, Clinical Affairs, America's Health Insurance Plans
- **Josephine P. Briggs**, director emeritus, National Center for Complementary and Integrative Health
- **Marc M. Boutin**, chief executive officer, National Health Council
- **Donna Cryer**, president & CEO of the Global Liver Institute
- **Craig Lipset**, head of clinical innovation, Global Product Development, Pfizer
- **Joanne Waldstreicher**, chief medical officer, Johnson & Johnson
- **Reed Tuckson**, managing director of Tuckson Health Connections

Under Construction: Reshaping and Focusing the Network



A national infrastructure for people-centered clinical research

20 Patient-Powered Research Networks + 13 Clinical Data Research Networks + 2 Health Plan Research Networks + 1 Coordinating Center



pcornet[®]

The National Patient-Centered Clinical Research Network