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Making Clinical Research All About Us

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pcornet The National Patient-Centered Clinical Research Network
Problems 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
- Too expensive
- Doesn't answer questions that matter most to people
- Too slow
- Data Not Shared
- Results not adopted
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?
Patient/Participant Recruitment

- Patient recruitment costs only account for 1.7 to 2.7 percent of overall costs across different clinical trial phases.
- Failed recruitment costs much, much more.
- Trials fail too often; avoidably and expensively
Most “data” about participant engagement in research is hypothetical.

Empirical evidence is from small, underpowered studies, usually in specific populations with specific interventions.

Lots of attention on patient/participant engagement. Need action.

Cochrane study shows insufficient evidence that limited tested methods work.
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PCORI and PCORnet have made a commitment to include patients at all stages.

Hard to do

Too often research designed by old white guys in old white lab coats

The first interaction with a patient should not be when she/he is asked to sign a consent.

What matters to you? How do we harvest that and integrate it into research design.
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Why Share?

- Enhances patient awareness and participation in trials
- Increases public information about marketed products
- Informs work of IRBs, policy makers, evidence based reviewers
- Mitigates bias in medical evidence base
- Informs design of future research and funding decision
- Increases public trust in research enterprise
- Meets ethical obligation to human subjects
Non-publication of Clinical Trial Results: NIH-funded Trials

- 68% published within 100 mos. of trial completion
- Less than 50% published within 30 mos. of trial completion

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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”
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

Demographics
- Death data

Diagnoses
- Medication orders

Procedures
- Labs

Geocodes

Tumor registry

Patient-generated data

Natural language processing-derived concepts

Social determinants of health

Genomic results

Biosamples

Claims

Genomic results

Patients-reported outcomes
Data queries in the PCORnet® distributed research network

PCORnet Coordinating Center

Query

Response

Question
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.

- Observational studies
- Health systems studies
- Interventional studies
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)
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)
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

- **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
PCORnet can conduct research on any health condition or specialty area, but it needs areas of initial specialization.

For each area of specialization:
- Expand and deepen data model
- Develop periodic population health reports
- Business development priorities and prospects
- Build and re-enforce clinical trial teams and infrastructure on the ground

Criteria for selecting the areas:
- High population impact
- Availability of expertise
- Sponsor interest
Improving Clinical Research

Idea | Application | Review | $$$$ | IRB | FDA | Enrollment and data collection | Results

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