Disruptive Thoughts

Practical Clinical Trials
Increasing the Value of Clinical Research for Decision Making in Clinical and Health Policy

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Decision makers in health care are increasingly interested in high-quality scientific evidence to support clinical and health policy decisions, however, the quality of available scientific evidence is often insufficient.

Thrombus Aspiration in ST-Elevation myocardial infarction in Scandinavia (TASTE trial). A multicenter, prospective, randomized, controlled clinical registry trial based on the Swedish angiography and angioplasty registry (SCAAR) platform. Study design and rationale

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What is PCORnet?
PCORnet’s goal

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.
Overall objectives of PCORnet: Achieving a single functional research network

- **Create** infrastructure, tools, and policies to support rapid, efficient comparative effectiveness research
- **Utilize** multiple electronic health records, insurance claims data, data reported directly by patients, and other data sources
- **Engage** patients, clinicians, and health system leaders throughout
- **Enable** external partners to collaborate with PCORI-funded networks
29 CDRN (11) and PPRN (18) awardees that include patients and systems in every state

This map depicts the number of PCORI-funded Patient-Powered or Clinical Data Research Networks that have coverage in each state.
Goals for each Clinical Data Research Network (CDRN)

🎯 Create a research-ready dataset of at least 1 million patients that is secure and comprehensive

🎯 Involve patients, clinicians, and health system leaders in all aspects of creating and running the network

🎯 Develop the ability to embed clinical trials into healthcare operations

🎯 Identify 3 cohorts of patients who have a condition in common and who can be characterized and surveyed
Goals for each Patient-Powered Research Network (PPRN)

- Establish patient population with a condition of interest (>50 patients for rare diseases; >50,000 for common conditions)
- Collect patient-reported data for ≥80% of patients
- Involve patients in network governance
- Create standardized research databases
Guiding principle: Make research easier

- Analysis ready data
  - Standard format
  - Harmonized definitions
  - Quality checked in advance

- Reusable analysis tools

- Efficient clinical trial enrollment and follow up mechanisms

- Simple, pragmatic studies integrated into routine care

- Administrative simplicity
Learning Health Care Systems and Pragmatic Trials

- Leverage available medical data from electronic health record (EHR) data to identify eligible patients
- Ascertain endpoints as part of routine healthcare delivery and administrative claims
- Simplify baseline and follow-up data collection through systematic direct patient contact (patient reported outcomes) and multiple data sources
- Large sample sizes embedded within healthcare systems and randomization provide large scale, limit selection biases and provide more generalizable results (by comorbidities, concomitant medication use, and sub-groups)