Healthcare Data Analytics: Classical Challenges, Contemporary Solutions

Sarah Greene, MPH
Associate Director, CER Methods & Infrastructure Program
Seattle Symposium on Healthcare Data Analytics

Patient-Centered Outcomes Research Institute

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Overview

About PCORI

PCORnet as a platform for observational and interventional studies

Paths forward
PCORI 101 (condensed version!)

PCORI is an independent non-profit funding agency, authorized through the Affordable Care Act

Fund clinical comparative effectiveness research (CER) that engages patients and other stakeholders in all aspects of the research process from inception of the project to dissemination of the results

Aims to answer real-world questions that matter to patients, their families and those who care for them, based on the patient’s specific circumstances and perspectives

“Based on my characteristics, what is the best choice for me?”
Our National Priorities for Research

- Assessment of Prevention, Diagnosis, and Treatment Options
- Improving Healthcare Systems
- Communication & Dissemination Research
- Addressing Disparities
- Accelerating PCOR and Methodological Research
Methodology = Key Focus at PCORI

Methodology Standards: 11 Broad Categories

- Formulating Research Questions
- Patient-Centeredness
- Data Integrity and Rigorous Analyses
- Preventing/Handling Missing Data
- Heterogeneity of Treatment Effects
- Data Networks
- Data Registries
- Adaptive and Bayesian Trial Designs
- Causal Inference
- Studies of Diagnostic Tests
- Systematic Reviews
PCORnet – PCORI’s Flagship Infrastructure Initiative

“Network of Networks”

Combines 11 Clinical Data Research Networks and 18 Patient-Powered Research Networks

Designed to leverage healthcare data from EHRs, patient-generated data, and even biospecimen data

Stands on shoulders of many progenitors including HMORN, Mini-Sentinel and other “big data” initiatives

Inaugural clinical trial “Optimal Maintenance Aspirin Dose for Patients with Coronary Artery Disease” commencing in 2015
Combining populations, patient engagement, and partners’ capabilities

PCORnet Infrastructure

Engage patients & clinicians in all aspects of research

Create efficient & effective processes for all aspects of conducting research

Include large, diverse populations from real-world care settings

Early Achievements:
- Integrating PROMIS into REDCAP
- Global Patient ID Techniques for De-duplication
- Novel Consent Strategies
Capacities will be in place to support all of these types of research.
Insights and Questions Prompted by the Symposium

- Enlightening to review the range of methodological challenges described during the symposium
  - Persistent issues in health/healthcare research that aren’t new or unique to these emergent large healthcare data sources
- Can large healthcare data sources be used to address these classical challenges (bias, misclassification, power, confounding)
- Could emergent designs and methods could leverage large healthcare data sources and help overcome the many challenges addressed during this symposium?
- How can we ensure this work reaches decision-makers?
Novel Designs as a (Partial) Solution for Pragmatic Trials

Adaptive Trial Designs – feasible in a learning healthcare system?
- Ideally, could get the best answer more quickly
- Yet new designs entail other new considerations
  - Operational issues embedding adaptive randomization at the point of care
  - Can we seamlessly integrate clinical trials and decision support?

Ethics and Regulatory Complexities for Pragmatic Clinical Trials

Nature of Interventions
Interventions that are the focus of CER and PCTs may present risks and consent issues similar to those in traditional clinical research; however, interventions directed at clinicians and patients can be categorically different. For example, testing a reminder system for clinicians or changing bathing procedures across hospital units involve interventions for which patients are not typically engaged directly. Instead, professionals are targeted for the intervention as mediator of risk, thereby complicating their ethical assessment. As these issues are addressed, it will be useful to assemble descriptions of interventions that others may learn from them.

Rethinking Randomized Clinical Trials for Comparative Effectiveness Research: The Need for Transformational Change
Bryan R. Luco, PhD, MBA; Judith A. Kramer, MD, MS; Steven N. Goodman, MD, MHS, PhD; Jason T. Connor, PhD; Sean Tunis, MD, MSc; Danielle Witcher, MHS; and J. Sanford Schwartz, MD

While advances in medical science have led to continued improvements in medical care and health outcomes, evidence of the comparative effectiveness of alternative management options remains inadequate for informed medical care and health policy decision making. The result is frequently suboptimal and inefficient care as well as unsustainable costs. To enhance or at least maintain quality of care as health reform and cost containment occurs, better evidence of comparative clinical and cost-effectiveness is required (1).
Data Credibility Still a Concern, Regardless of Design

- Data provenance issues
- EHRs do not produce research ready data in their native form
- Existential questions about “missing” data
- If we are using research data to influence practice, decision-makers need reassurance that our conclusions are valid
- Patients and clinicians also deserve that same reassurance
  - Frustration and skepticism may result when new published studies contradict previous research
Patient Data

Data get better with use!
A driving force in the effort to make patients equal partners in designing clinical trials is a nonprofit group called the Patient-Centered Outcomes Research Institute, or PCORI, which was established by the federal Affordable Care Act. PCORI, funded from a fee on health plans, was set up to do comparative effectiveness research. The group requires that trials it funds include patients in trial design and other aspects of the process.

“What patients really bring is the relevance of the research. Is this really meaningful to the patient community?” says Susan Sheridan, director of patient engagement at PCORI.

Karen Wernli, assistant investigator at Group Health Research Institute in Seattle, created a dictionary of scientific and technical terms for patients who are advisers on an observational study comparing the effectiveness of MRIs versus mammograms in early detection of cancer in women after breast-cancer treatment. Patients helped choose the outcomes of the study and are helping develop a decision aid for doctors and patients to use to determine which method works best for each individual.
Considerations for work in healthcare data analytics

- Data provenance
- Study design
- Study methods

AND

- Data privacy
- Dissemination
- Reproducibility

Confronting statistical challenges of using electronic health record data to conduct health research
Keep PCORI CER Methods Funding Opportunities in Mind!

Improving Methods for Conducting Patient-Centered Outcomes Research - Fall 2014 Cycle

In this PFA, we seek projects to address gaps in methodological research relevant to conducting PCOR. Results of these projects will inform future iterations of PCORI’s Methodology Report. The improvement of existing methods will benefit all stakeholders, including researchers planning investigations, policy makers weighing the value of healthcare interventions; and patients, clinicians, and caregivers facing healthcare decisions.

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Thank you!

sgreene@pcori.org
www.pcori.org