Uncertain Times Call for Empirical Bioethics: Using Qualitative and Quantitative Research Methods to Inform Debate about Patient-Centered Outcomes Research

Stephanie Kraft
Stephanie Morain
Emily Largent
Jeremy Sugarman

Overview

- Setting the context
- Recent empirical projects
  - Stephanie Kraft
  - Stephanie Morain
  - Emily Largent
- Discussion
  - Reflect on the contributions—and limitations—of different empirical approaches
  - Identify common themes from existing data
  - Suggest strategies to guide future empirical research

Disclosure

- I co-chair the Ethics and Regulatory Core for the NIH Health Care Systems Research Collaboratory and co-led the Ethics and Regulatory Task Force for PCORNet
- I receive(d) salary/grant support through Johns Hopkins University for this work
- The views expressed here are my own and do not necessarily reflect the views of the sponsors, the Collaboratory or PCORNet

Background Conditions

- Broad moral claim to obtain evidence to improve clinical practice since most decisions are now made without reliable evidence to know which choices optimize health
- Technology permits conducting large scale research and cohort finding for rare diseases and special populations, often with minimal incremental risks and burdens and less cost
- Patients and communities are increasingly engaged in research

4

Types of Trials

- Explanatory
  - "primarily designed to determine the effects of an intervention under ideal circumstances"
- Pragmatic
  - "primarily designed to determine the effects of an intervention under the usual conditions in which it will be applied"

Thorpe KE, et al. *J Clin Epidem* 2009; 62: 464-475

Attributes of PCTs

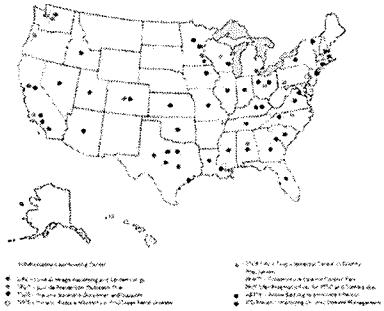
1) an intent to inform decision-makers (patients, clinicians, administrators, and policy makers), as opposed to elucidating a biological or social mechanism;
2) an intent to enroll a population relevant to the decision in practice and representative of the patients/populations and clinical settings for whom the decision is relevant;
3) a focus on outcomes of relevance to patients and clinicians; and
4) either an intent to
   (a) streamline unnecessary procedures and data collection so that the trial can focus on adequate power for informing the clinical and policy decisions targeted by the trial or
   (b) measure a broad range of outcomes.

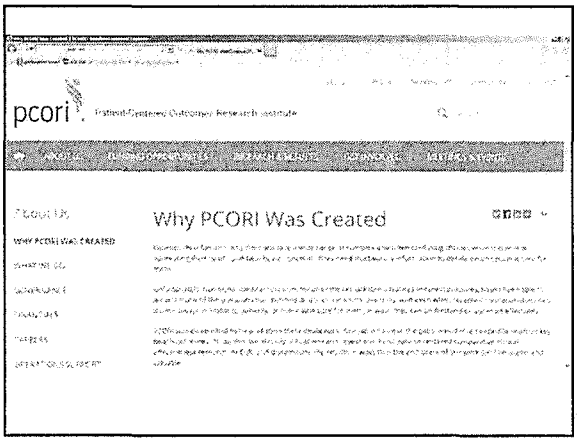
Califf RM, Sugarman J. *Clin Trials* 2015.

NIH Health Care Systems Research Collaboratory

- Pragmatic trial design
- Electronic health record as core data collection instrument
- At least 2 integrated health systems collaborating
- 10 demonstration projects

NIH Health Care Systems Research Collaboratory





Disease Based Activism

- Enhance access to experimental treatments
- Shape the research process through involvement

Types of Community Engagement

- Advocates
- Community Advisory Boards (CABs)
- 'Community' members on IRBs
- Community consultation
- Community preparedness
- Community permission
- Community-based research
- Community-based participatory research

Patient Centered Outcomes Research

... helps people and their caregivers communicate and make informed healthcare decisions, allowing their voices to be heard in assessing the value of healthcare options. This research answers patient-centered questions, such as:

1. "Given my personal characteristics, conditions, and preferences, what should I expect will happen to me?"
2. "What are my options, and what are the potential benefits and harms of those options?"
3. "What can I do to improve the outcomes that are most important to me?"
4. "How can clinicians and the care delivery systems they work in help me make the best decisions about my health and health care?"

http://www.pcori.org/content/patient-centered-outcomes-research

PCOR: Patients as Collaborators

- Research development
- Conduct
- Oversight

An Ethics Framework for a Learning Health Care System: *A Departure from Traditional Research Ethics and Clinical Ethics*

Hastings Cent Rep 2013 Jan/Feb:S16

JAMA

Sugarman J, Califf RM. Ethics and regulatory complexities for pragmatic clinical trials. *JAMA* 2014; 311: 2381-2382.

CLINICAL TRIALS

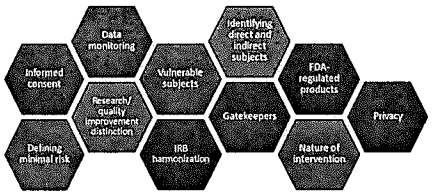
Anderson M, Califf R, Sugarman J, for the NIH Health Care Systems Research Collaboratory Cluster Randomized Trial Workshop. Ethical and regulatory issues of pragmatic cluster randomized trials in contemporary health systems. *Clin Trials* 2015; 12: 276-286.

CLINICAL TRIALS

SCT

Clinical Trials Special Series

Guest Editors: Jeremy Sugarman and Robert Califf



http://ctj.sagepub.com/content/early/recent

NIH Ethics Supplements

- CTSA (University of Washington & Stanford)
- NIH Collaboratory
  - TiME (University of Pennsylvania)
  - ABATE (University of California - Irving)
  - Coordinating Center (Duke & Hopkins)

Concluding Comments

- Addressing the ethical and regulatory challenges of PCTs is surprisingly complex
- The well-rehearsed habit of 'adding protections' in research ethics *may not* be actually be providing needed protections and may inadvertently stymie important research efforts
- Additional data and deliberation should help inform the development of appropriate policies and procedures