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

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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
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- The views expressed here are my own and do not necessarily reflect the views of the sponsors, the Collaboratory or PCORNet

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

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

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”


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.

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

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

Disease Based Activism

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

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

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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

Clinical Trials Special Series
Guest Editors: Jeremy Sugarman and Robert Califf

NIH Ethics Supplements
CTSA
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http://ctj.sagepub.com/content/early/recent

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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