Lingering and Emerging Ethical Issues for Pragmatic Clinical Trials and Learning Health Systems

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Overview

• Conceptual work
• SUPPORT
• Basic ethical issues in PCTs
• Signals from empirical research
• Emerging issues

Disclosure

• I am a member of Merck KGaA’s Bioethics Advisory Panel and Stem Cell Research Oversight Committee; and a member of IQVIA’s (formerly Quintiles) Ethics Advisory Panel
• 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 PCORNNet
• 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 PCORNNet

Disclaimer

• Given the brevity of this talk, my hope is to simply sketch some of the major ethical issues related to PCTs and LHSs
• I have selected prominent examples to illustrate particular points and trigger discussion; it would be inappropriate to consider this to represent a comprehensive review
Alternative Bioethical Views

IOM/NAM Workshop

Major Areas of Controversy
- Consent
- Risks and benefits
- Standard of care

Background Conditions
- There is a 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

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 + 5 new projects approved for a planning phase

Public Hearing
Ethics and Regulatory Issues in the Collaboratory

- Multi-stakeholder conversations at the planning stage convened by the Ethics & Regulatory Core
  - Investigators and research teams
  - Sponsors
  - IRBs
  - Regulators
- Minutes reviewed and posted
- Updates following project implementation

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**Clinical Trials Special Series**

Guest Editors: Jeremy Sugarman and Robert Califf

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Signals from Empirical Research

- At least a substantial minority of people want to be meaningfully engaged in research decision-making
  - Regardless of risk
  - Regardless of health care norms
- It is unclear if the nature of these activities were clearly understood and their best interests were not compromised
- Since requiring traditional written consent may compromise some research this issue must be better understood

Emerging Issues

- Standards for data monitoring
- Patients in 'non-traditional' roles
- Incidental findings

Concluding Comments

- There is an array of ethical and regulatory challenges for PCTs and CER
- Substantial empirical and conceptual attention directed at these issues is needed before the possibility of achieving a learning health system can be realized
- To be most meaningful, these efforts should be grounded in actual experiences