

# Learning Healthcare, Comparative Effectiveness, and Ethics

## The Common Good Framework

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Issues in Human Subjects  
Protections

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# Topics for this Morning

- Today: Segregation Model of Research and Practice
- Tomorrow: Integration Model- Learning Health Care Systems
- Comparative Effectiveness Research
- Ethics of Learning Healthcare Systems
- Cancer Case Example



# Project Team

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# Segregation Model of Research and Practice

- **1960s-1970s: The influence of “research scandals”**
- **1973-74: Federal investigations and regulations**
  - Required IRB review
  - Required informed consent
- **Regulations needed to be able to distinguish clinical research from clinical practice**
  - Prospective ethical oversight was needed for clinical research, while not needed for clinical practice



# Problems with Segregation Model

- **Over-protection (of low risk research)**
- **Under-protection (from unsafe or unproven care)**
  - Several hundred thousand people die needlessly each year from medical mistakes
  - Countless patients get the wrong care or too much care for lack of evidence



# Integration Model of Research and Practice: Learning Healthcare System

- **A system in which “knowledge is so embedded into the core of the practice of medicine that it is a natural outgrowth and product of the health care delivery process and leads to continual improvement in care.”**

Institute of Medicine (US) Roundtable on Evidence-Based Medicine; Olsen LA, Aisner D, McGinnis JM, editors. The Learning Healthcare System: Workshop Summary. Washington (DC): National Academies Press (US); 2007.



# Some Features of a Learning Healthcare System

- **Knowledge generation from every clinical encounter**
- **That learning is translated back into improved care in the same settings**
- **Delivering care, and learning systematically and rigorously learning from that care, are thus simultaneous commitments of LHCS**
- **Continuous (integrated) learning to discover**
  - How can patient safety be improved?
  - Among existing clinical options, what works best for which patients? (comparative effectiveness research)



# Comparative Effectiveness Research

- **“the generation and synthesis of evidence that compares the benefits and harms of alternative methods to prevent, diagnose, treat and monitor a clinical condition or to improve the delivery of care.”**

*Committee on Comparative Effectiveness Research Prioritization of the Institute of Medicine. Initial national priorities for comparative effectiveness research. Washington, DC 2009*

- **Focus on existing, approved and widely used interventions- low risk, more routine**
- **Evidence helps patients and clinicians make more informed decisions that improve care**



# Comparative Effectiveness Research Designs

- **Observational designs**
  - No aspect of clinical care is altered or changed
  - Retrospective using only existing patient data/electronic health records
  - Prospective with existing and/or new data
- **Randomized designs**
  - Some aspect of clinical care is determined by protocol
  - Practical or pragmatic trials
  - Classic clinical trials
  - Cluster or individual



# Common Purpose Framework for Learning Healthcare Systems

- **Respect the rights and dignity of patients**
- **Respect clinician judgments**
- **Provide optimal clinical care to patients**
- **Avoid imposing nonclinical burdens and risks**
- **Address health inequalities**
- **Participate in the learning process (clinicians)**
- **Participate in the learning process (patients)**

Faden, R., Kass, N., Goodman, S., Pronovost, P., Tunis, S., Beauchamp, T. "An Ethics Framework for a Learning Health Care System: A Departure from Traditional Research Ethics and Clinical Ethics," *Hastings Center Report*. 2013 Jan-Feb; Spec No:S16-27.



# Implementing the Common Good Framework

- **Engagement:** Of patients in setting CER priorities and in the ethics oversight process of studies, including in determining which disclosure and consent practices are appropriate for particular studies
- **Transparency:** To the patient community- what learning activities are currently underway, aims, types of patients, and effects on medical care
- **Accountability:** To the patient community- how health care delivered in their healthcare setting is, or is not changing, as a result of what is being learned



# Example from Oncology

- **“In 1996, the Eastern Cooperative Oncology Group (ECOG) reported results of a phase III trial comparing four different platinum- based doublet chemotherapy regimens that had been developed for treatment of advanced non–small-cell lung cancer (NSCLC). The ECOG study randomly assigned more than 1,200 patients to receive cisplatin/paclitaxel, cisplatin/gemcitabine, cisplatin/docetaxel, and carboplatin/paclitaxel....”**

Hahn, Olwen M., and Richard L. Schilsky. "Randomized Controlled Trials and Comparative Effectiveness Research." *Journal of Clinical Oncology* 30.34 (2012): 4194-4201.



# Assumptions

- **These different NSCLC chemotherapy regimens are all approved, in wide use and considered comparable in effectiveness and side effect profile (in the absence of data)**
- **Learning healthcare system with ethics framework**
- **Engagement, transparency, accountability**



# Disclosure-Authorization Options

**Consider two CER studies: both use electronic health record data to compare outcomes for patients on the different regimens**

**#A: Observational study-** physicians and patients choose regimen

**#B: Randomized study-** initial regimen assigned by protocol

## Options

- 1. Transparency to patient community**
- 2. Patients informed- opt out**
- 3. Patients informed- opt in**