Topic 6: Measuring Outcomes

Rachel Richesson, PhD
Duke University School of Nursing
Lesley Curtis, PhD
Duke Clinical Research Institute
Outline

• Definitions
• The electronic data puzzle
• Caveats for EHR data in research
• Possible sources of error
• Data quality assessment recommendations
• Clinical phenotypes
• Reporting guidelines for PCTs
• Patient-reported outcomes
• Conclusions & recommendations
Outcomes vs endpoints

- Direct
- Surrogate
- Composite
Where is the signal?

- EHR (laboratory values, treatments, etc)
- Claims data (does the event generate a bill?)
Reality is not straightforward

Source: Greg Simon, MD, Group Health Research Institute
Data sources for endpoints in PCTs

“The first challenge in using big biomedical data effectively is to identify what the potential sources of health care information are and to determine the value of linking these together.”

Finding the Missing Link for Big Biomedical Data
(See Figure 1)

Griffin M. Weber, MD; Kenneth D. Mandl, MD, MPH; Isaac S. Kohane, MD, PhD

Data sources for endpoints in ePCTs

• EHR or ancillary health information systems
• Patient report
• Patient measurement
Caveats when using EHR data for endpoints (selected)

- Data may be transformed/coded for purposes other than research & clinical care

Brad Hammill, unpublished
Caveats when using EHR data for research (selected)

- Data captured in clinical notes may not be available
- EHRs are often highly customized
- EHRs may present multiple sources of similar data

Source: Hersh WR et al. Med Care 2013;51:S30-S37.
Caveats when using EHR data for research (selected)

“EHRs may present multiple sources of data that affect data provenance.”

Caveats for the Use of Operational Electronic Health Record Data in Comparative Effectiveness Research (See Figure 1)

William R. Hersh, MD, Mark G. Weiner, MD, Peter J. Embi, MD, MS, Judith R. Logan, MD, MS, Philip R.O. Payne, PhD, Elmer V. Bernstam, MD, MSE, Harold P. Lehmann, MD, PhD, George Hripcsak, MD, MS, Timothy H. Hartzog, MD, James J. Cimino, MD, and Joel H. Saltz, MD, PhD

Med Care. 2013 Aug; 51(8 0 3): S30–S37
doi: 10.1097/MLR.0b013e31829b1dbd
Caveats when using EHR data for research (selected)

- EHRs often do not tell a complete story

Source: Hersh WR et al. Med Care 2013;51:S30-S37.
Longitudinal data linkage

• To fully capture *all* care—complete longitudinal data—linking research & insurance claims data is often necessary

• Without explicit consent, getting longitudinal data from an insurance carrier can be an insurmountable hurdle, both technically and legally
Enabling pragmatic research: escreening, eenrollment & efollow-up

- Call FOLLOW-UP
  - Patient Reported Outcomes
  - Medication use
  - Health outcomes

- Portal FOLLOW-UP
  - Patient Reported Outcomes
  - Medication use
  - Health outcomes

- PCORNet Coordinating Center FOLLOW-UP
  - Via Common Data Model
  - Longitudinal health outcomes

- CMS, Payer FOLLOW-UP
  - Longitudinal health outcomes

ADAPTABLE Enrollee

Baseline Data

OR

Adaptable
The Aspirin Study
Choosing and specifying endpoints in ePCTs

• Endpoints and outcomes need to be available as part of routine care

**Easy**
- Acute MI
- Broken bone
- Hospitalization

**Hard**
- Suicide attempts
- Gout flares
- Silent MI
- Early miscarriage
Key questions for choosing endpoints

Is the outcome medically significant such that a patient would seek care?

- Does it require hospitalization?
- Will the endpoint be medically attended?
- Is the treatment generally provided in inpatient or outpatient settings?
Endpoints in ePCTs

• Endpoints should be meaningful to providers and patients
  • MACE vs myocardial infarction
  • Good example of a blood test vs a clinical event
• More pragmatic endpoints …
  • Matter to providers and patients
  • Are captured reliably as part of routine clinical care
  • Do not require central adjudication
  • Are shorter-term in nature

Choosing an endpoint that is not captured reliably as part of routine clinical care or impedes the clinical workflow is not pragmatic!
Data is a surrogate for clinical phenomena

Error Impact on Trials

Truth
Health status of the patient

Concept
Clinician or patient’s conception

Record
EHR/PHR

Read
2ⁿ clinician’s or researcher’s conception

Model
Computable representation

Observation & interpret

Document

Error

Error

Implicit

Process
Key questions for using EHR data

What is the phenomenon you are trying to identify or measure?

In what type of health care activity, event, documentation or data value could a “signal” be detected?

What are the sources of error?

How can you assess and reduce that error?
Data quality assessment

• Identify variation between populations at different sites or study groups

• Recommend formal assessment of accuracy, completeness & consistency for key data

• Data quality should be described, reported & informed by workflows
Case example: Collaborative Care for Chronic Pain in Primary Care (PPACT)

PROs were needed, but were not standardly collected across diverse regions.
Case example: PPACT

- Project leadership worked with national Kaiser to create buy-in for a common instrument
- Local IT built it within each region
- A multi-tiered approach supplemented the clinically collected PRO data at 3, 6, 9, 12 months
- A follow-up phone call by research staff was necessary to maximize data collection at each time point
Outcomes measured via direct patient report

- Patient-reported outcomes (PROs) often best way to measure quality-of-life
- Challenges
  - Not routinely & consistently used in clinical care
  - Not regularly recorded in EHR
- Need mechanism to collect PROs
Mobile devices

- Smartphones, tablet computers, and portable, implantable, or wearable medical devices (mHealth)
  - Some mHealth devices transmit data to a data warehouse every night
  - Largely considered imperfect measures
- Patient-facing mobile phone apps can be used in PCT for passive or active surveillance
Administration

AHRQ Challenge to Focus on PROs in EHRs

According to a Federal Register notice on December 26, 2017, AHRQ is going to develop user-friendly technical tools to collect and integrate patient-reported outcome data in electronic health records or other health information technology products. AHRQ says that “the limited inclusion of PRO data in electronic health records (EHRs) and other health information technology (IT) solutions reduces the understanding and use of the patient's perspective in research and clinical care… Moreover, standards do not exist for collecting and integrating PRO data into health IT systems, thereby limiting the ability to easily share these data across health systems for research or other purposes including quality improvement.” Mark your calendars, as AHRQ has targeted the Fall of 2018 for these challenges.
Consider the reporting guidelines when choosing outcomes

- Clearly define primary & secondary outcome measures
- Report methods used to enhance the quality of measurements
- Explain how selected outcomes & length of follow-up are important to stakeholders
Defining outcomes with clinical phenotypes

“Differences across phenotype definitions can potentially affect their application in healthcare organizations and the subsequent interpretation of data.”

A comparison of phenotype definitions for diabetes mellitus
(See Figure 1 and Table 1)

Rachel L Richesson, Shelley A Rusincovitch, Douglas Wixted, Bryan C Batch, Mark N Feinglos, Marie Lynn Miranda, W Ed Hammond, Robert M Califf, Susan E Spratt

J Am Med Inform Assoc, Volume 20, Issue e2, 1 December 2013, Pages e319–e326; doi.org/10.1136/amiajnl-2013-001952
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Perspective

Pragmatic (trial) informatics: a perspective from the NIH Health Care Systems Research Collaboratory

Rachel L Richesson,1,2 Beverly B Green,3 Reesa Laws,4 Jon Puro,5 Michael G Kahn,6 Alan Bauck,4 Michelle Smerek,7 Erik G Van Eaton,8 Meredith Zozus,9 W Ed Hammond,2 Kari A Stephens,10 and Greg E Simon3

• Competition for IT resources
• Need to optimize clinical data for research
• Only small proportion of research in EHRs

• Need to capture intervention or control activities
• Including standard of care
• Need to enable learning & research activities into EHR functions

https://academic.oup.com/jamia/article/24/5/996/3069877/Pragmatic-trial-informatics-a-perspective-from-the
Important things to know

- Endpoints and outcomes should be meaningful to providers and patients.
- Endpoints and outcomes should be relatively easy to collect (i.e., pragmatic).
- Researchers do not control the design or data collected in EHR systems.
- Good practices for using clinical data in PCTs are based upon scientific principles.
Very important …

- The data available from the EHR may be convenient & pragmatic, but might not actually drive clinical practice or policy if used as endpoints.

- Need to make sure that the endpoint that IS conveniently available will also be accepted as one that will be influential for stakeholders when the PCT results are disseminated.

- “Plan with Implementation in Mind” (Topic 3)
Important things to do

• Ask questions that the data will support and design trials to minimize new data collection
• Engage EHR and data experts when defining endpoints and outcomes
• Budget for data and systems experts at each site (... and then double it)
• Develop a robust data quality assessment plan to improve value of data and to detect and address data issues
1. What is your primary endpoint?
2. Is that endpoint sufficiently informative for your stakeholders?
3. What challenges do you anticipate in trying to ascertain that endpoint?
4. How might you address those challenges?