Measuring Outcomes

Emily C. O'Brien, PhD

Associate Professor of Population Health Sciences

Duke University

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Learning goals

- Describe methods for measuring outcomes using data sources such as electronic health records (EHRs) and patient-reported outcomes (PROs)
- Discuss the integration of a health equity lens in evaluating outcomes
- Q & A with attendees

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Endpoints and outcomes

- An endpoint usually refers to an analyzed parameter (such as change from baseline at 6 weeks in mean PROMIS Fatigue score)

- An outcome usually refers to a measured variable (such as peak volume of oxygen or PROMIS Fatigue score)

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Important things to know 👓

- Endpoints and outcomes should be meaningful to providers and patients

- Endpoints and outcomes should be relatively easy to collect (ie, pragmatic)

- Researchers do not control the design or data collected in EHR systems

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Choosing and specifying ePCT endpoints

Endpoints and outcomes should be available as part of routine care

Easy

- Acute MI
- Broken bone
- Hospitalization

Hard

- Suicide attempts
- Gout flares
- Silent MI
- Early miscarriage

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Key questions for <u>choosing</u> 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?

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Data sources for endpoints in ePCTs

"The first challenge in using big biomedical data effectively is to identify what <u>the potential sources of health care information are</u> and to determine the value of linking these together."
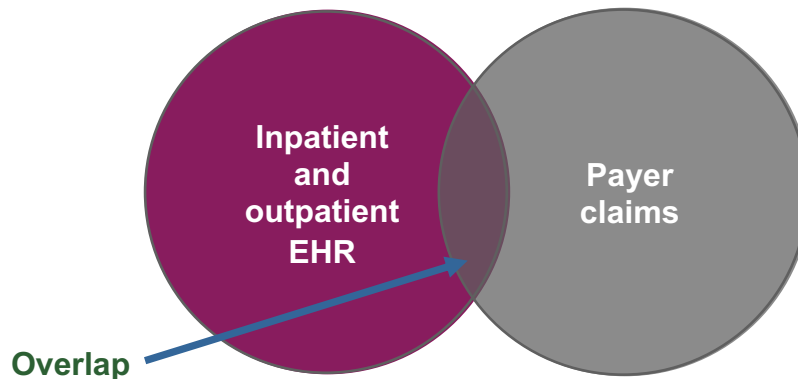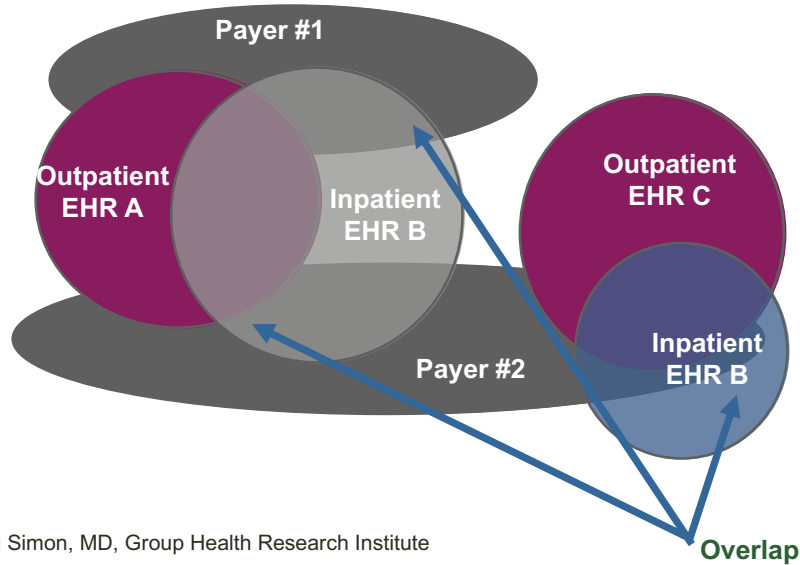
Weber GM et al. JAMA. 2014;311(24):2479-2480.

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Where is the signal?

- EHR (laboratory values, treatments, etc)
- Claims data (does the event generate a bill?)

Inpatient and outpatient EHR

Payer claims

Overlap

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Reality is not straightforward

Payer #1

Outpatient EHR A

Inpatient EHR B

Outpatient EHR C

Inpatient EHR B

Payer #2

Overlap

Source: Greg Simon, MD, Group Health Research Institute

Longitudinal data linkage

- To fully capture all care—complete longitudinal data— linking research and 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

Data sources for endpoints in ePCTs

- EHR or ancillary health information systems
- Patient report
- Patient measurement

It's a balancing act

High relevance to real-world decision-making may come at the expense of efficiency

For example, a trial measuring outcomes that matter most to patients and health systems may not be able to rely exclusively on information from the EHR, and instead need to assess patient-reported outcomes, which is more expensive and less efficient

Outcomes measured via direct patient report

- PROs are often the best way to measure quality of life
- Challenges
  - Not routinely or consistently used in clinical care
  - Not regularly recorded in EHR
- Need a mechanism to collect PROs

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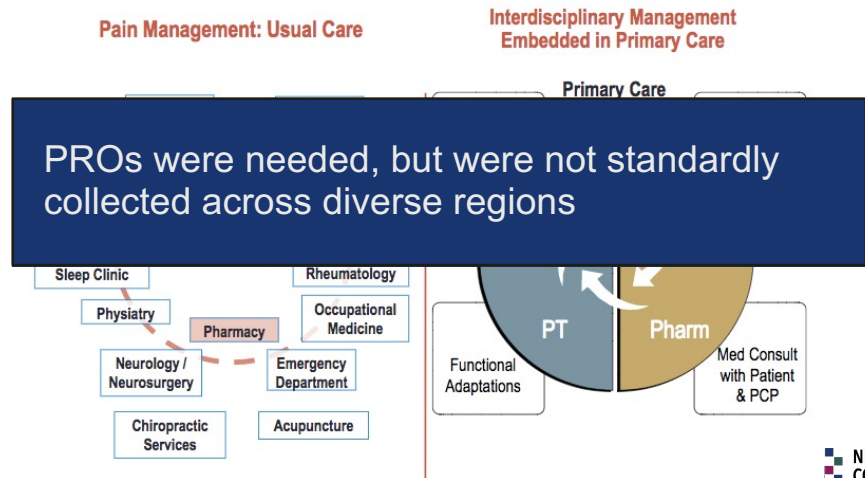Rethinking Clinical Trials®
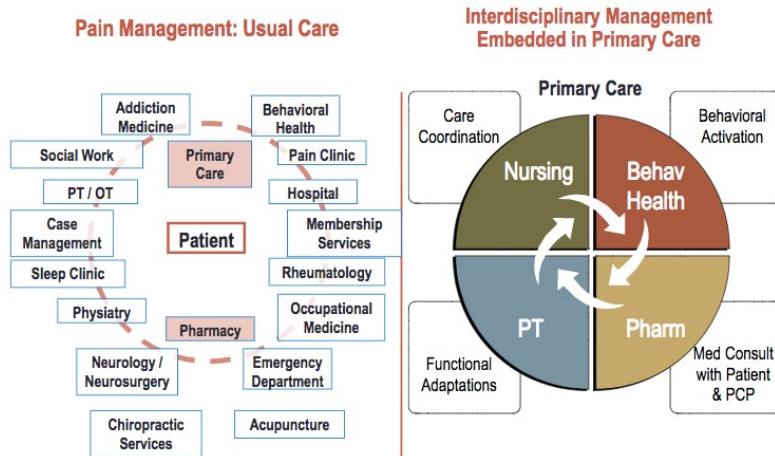
Case example: Collaborative Care for Chronic Pain in Primary Care (PPACT)

Source: Lynn DeBar, PhD, MPH, Kaiser Permanente Washington Health Research Institute

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Case example: Collaborative Care for Chronic Pain in Primary Care (PPACT)

Pain Management: Usual Care

Interdisciplinary Management Embedded in Primary Care

Primary Care

> PROs were needed, but were not standardly collected across diverse regions

Sleep Clinic

Rheumatology

Physiatry

Occupational Medicine

Pharmacy

Neurology / Neurosurgery

Emergency Department

Chiropractic Services

Acupuncture

PT

Pharm

Functional Adaptations

Med Consult with Patient & PCP

Source: Lynn DeBar, PhD, MPH, Kaiser Permanente Washington Health Research Institute

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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 multitiered approach supplemented the clinically collected PRO data at 3, 6, 9, and 12 months
- A follow-up phone call by research staff was necessary to maximize data collection at each time point

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Enabling pragmatic research:
e-screening, e-enrollment & e-follow-up
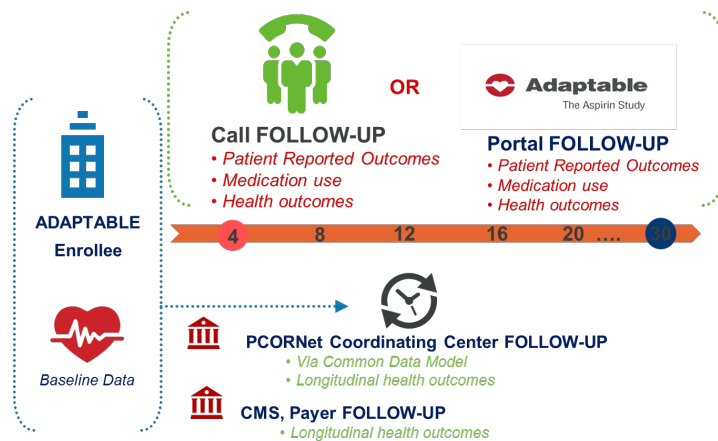
Mobile devices for outcome measurement

- 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 ePCTs for passive or active surveillance

Data is a surrogate for clinical phenomena

Error Impact on Trials
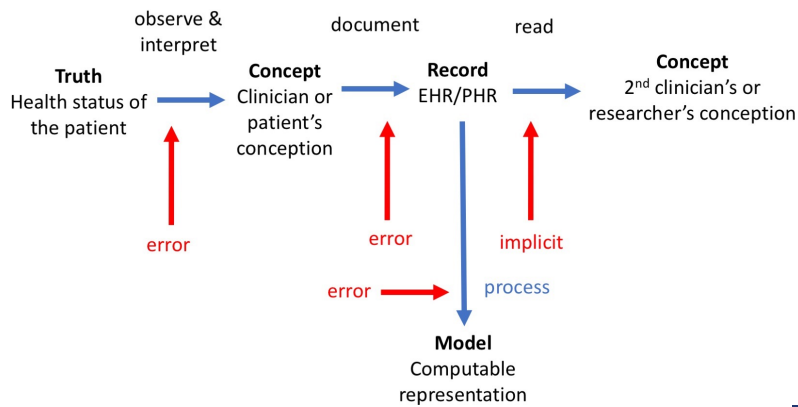
Adapted from Hripcsak et al 2009

Data quality assessment

- Identify variation between populations at different sites or study groups
- Recommend formal assessment of accuracy, completeness, and consistency for key data
- Data quality should be described, reported, and informed by workflows

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

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Concluding points

- Data available from the EHR may be convenient and pragmatic, but might <u>not</u> actually drive clinical practice or policy if used as endpoints
- Need to make sure that conveniently available endpoint <u>will also be accepted</u> as influential for stakeholders when the ePCT results are disseminated
- Plan with implementation in mind

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