Measuring Outcomes

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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

Outcome, Measure, Endpoint

- An outcome usually refers to a variable of interest or a meaningful aspect of health (such as oxygen volume or fatigue).
- A measure usually refers to a specific and standardized process to obtain information on an outcome.
  - Includes: instructions, administration materials, content, formatting, and scoring rules.

Types of measures

Patient-reported outcome measures (PROM)

Observer-reported outcome measures (ObsRO)

Clinician-reported outcome measures (ClinRO)

Performance outcome measures (PerfO)

Outcome, Measure, Endpoint

- An endpoint usually refers to a precisely defined variable that is statistically analyzed to address a particular research question.

Example:

- Change from baseline at 6 weeks in mean PROMIS Fatigue score.
- Mean differences in PROMIS Fatigue scores between patients in treatment and standard of care groups, after controlling for baseline status.

Important things to know

- Outcomes and their related endpoints should be meaningful to providers and patients
- Outcomes and related measures should be relatively easy to collect (i.e., pragmatic)
- Researchers do not control the design or data collected in EHR systems

Choosing and specifying ePCT endpoints

Outcomes and their related endpoints should 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 <u>choosing</u> endpoints

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

Does it require hospitalization?

Will the event be medically attended?

Is the treatment generally provided in inpatient or outpatient settings?

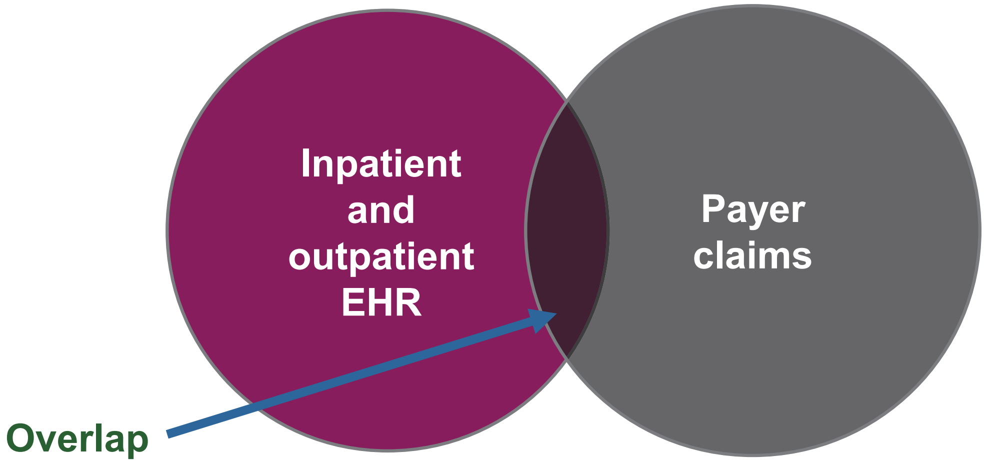
Data sources for endpoints in ePCTs

> “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.”

Weber GM et al. JAMA. 2014;311(24):2479-2480.

Where is the signal?

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

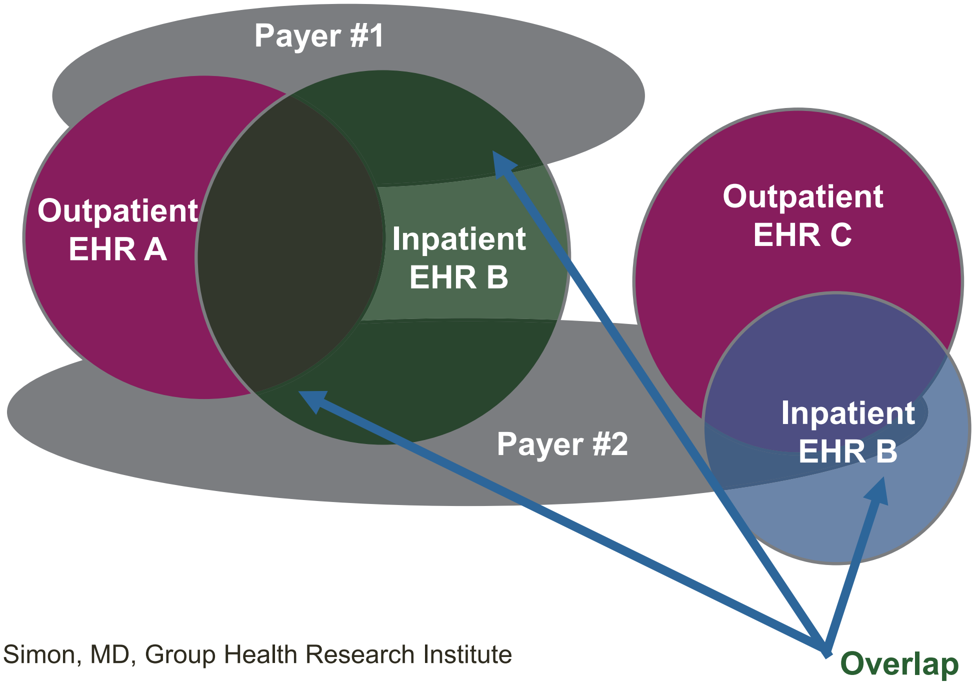


Inpatient and outpatient EHR

Payer claims

Overlap

Reality is not straightforward



Payer #1

Outpatient EHR A

Inpatient EHR B

Payer #2

Outpatient EHR C

Inpatient EHR B

Overlap

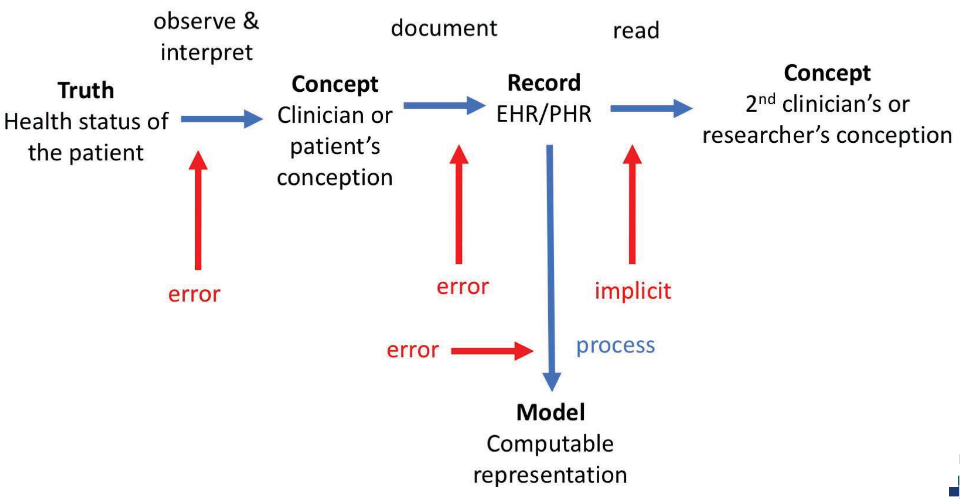
Source: Greg Simon, MD, Group Health Research Institute

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 is a surrogate for clinical phenomena

Error Impact on Trials



observe & interpret

document

read

Truth
Health status of the patient

Concept
Clinician or patient's conception

Record
EHR/PHR

Concept
2nd clinician's or researcher's conception

error

error

implicit

error

process

Model
Computable representation

Adapted from Hripcsak et al 2009

Data sources for endpoints in ePCTs

Traditional:

- EHR or ancillary health information systems

Complementary:

Other types of health data not routinely collected outside of standard clinical practice:

- Patient reported data

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

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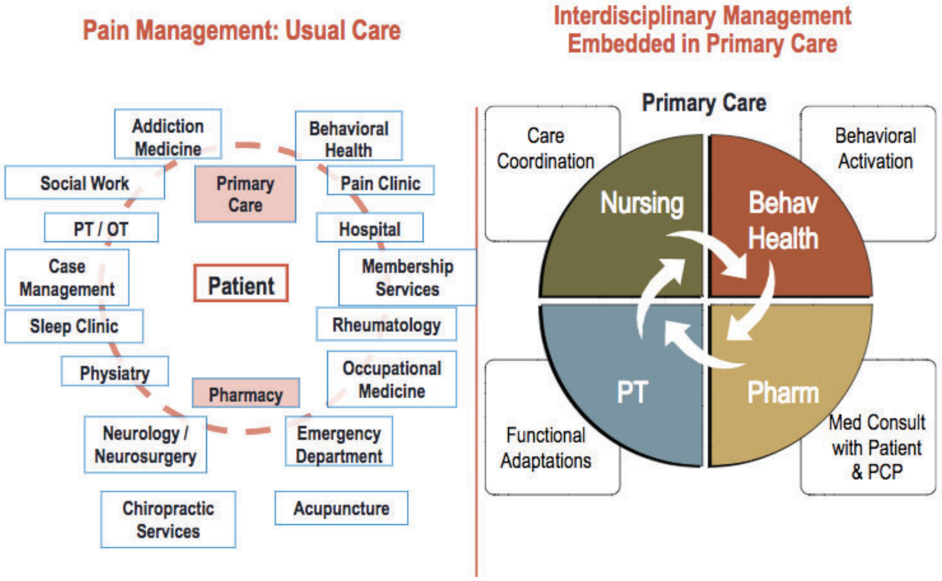
Outcomes measured via direct patient report

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

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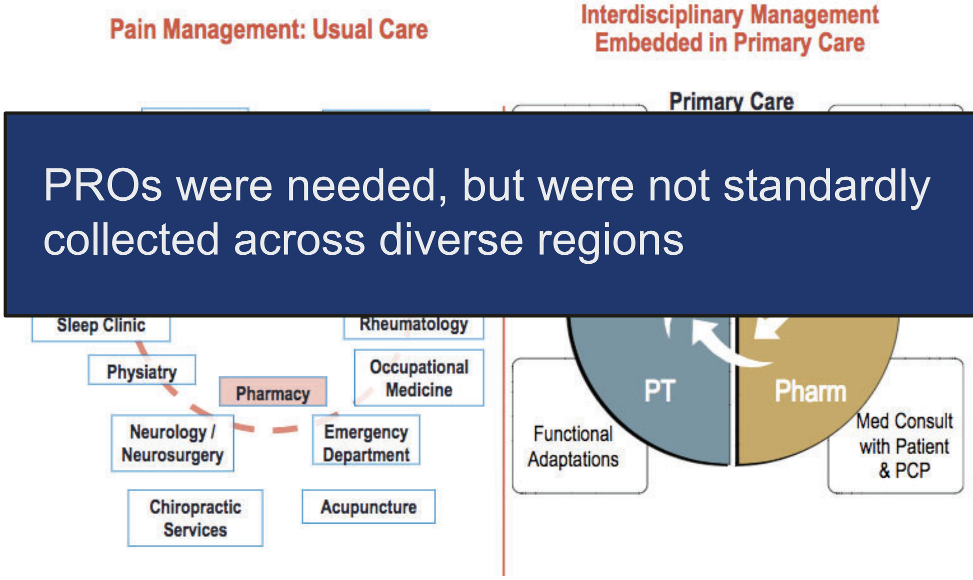
Case example: Collaborative Care for Chronic Pain in Primary Care (PPACT)



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

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 PROM 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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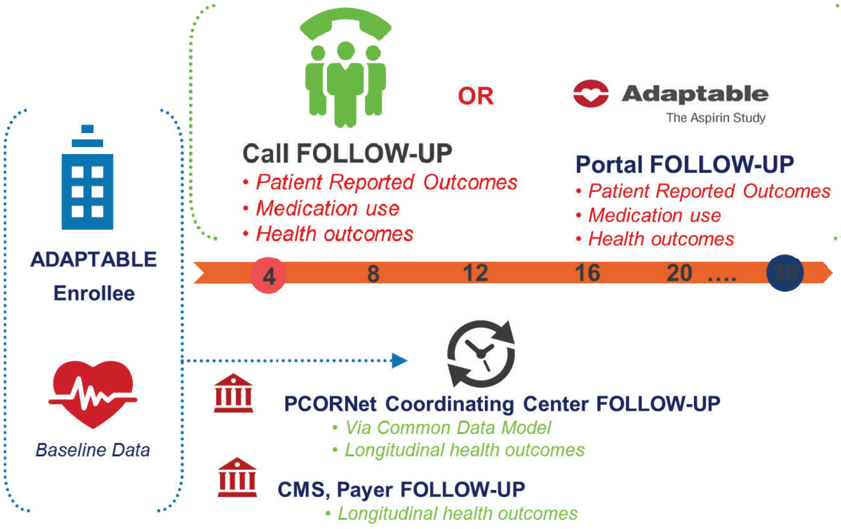
Enabling pragmatic research: e-screening, e-enrollment & e-follow-up



ADAPTABLE Enrollee

Baseline Data

OR

Adaptable
The Aspirin Study

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*

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

A Health Equity Lens

J Am Med Inform Assoc. 2023 Sep; 30(9): 1561–1566.
Published online 2023 Jun 26. doi: 10.1093/jamia/ocad115

PMCID: PMC10436149
PMID: 37364017

Potential bias and lack of generalizability in electronic health record data: reflections on health equity from the National Institutes of Health Pragmatic Trials Collaboratory

Andrew D Boyd, Rosa Gonzalez-Guarda, Katharine Lawrence, Crystal L Patil,

- “As the number of ePCTs using EHR-derived data grows, so does the risk that research will become more vulnerable to biases due to differences in data capture and access to care for different subsets of the population, thereby propagating inequities in health and the healthcare system”
- **Challenges:**

Data loss due to variable use of technology

Incomplete and variable capture of data on social determinants of health

Lack of representation from vulnerable populations that do not access or receive treatment

A Health Equity Lens

Contemporary Clinical Trials

Volume 130, July 2023, 107238

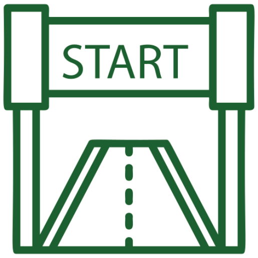
Short Communication

Equity and bias in electronic health records data

Andrew D. Boyd[a], Rosa Gonzalez-Guarda[b], Katharine Lawrence[c],

Recommendations to reduce bias:

- Collect demographic and social determinants of health
- Evaluate and address data collection barriers across diverse populations
- Utilize community-engaged approaches
- Evaluate the reading level of all patient-facing data collection tools (e.g. PROMs) and consider translation/cross-cultural validation



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

Always!

Important things to do

- Ask questions that the data will support
- Design trials to minimize new data collection
- Talk to patients and stakeholders when identifying outcomes
- Engage EHR and data experts when defining endpoints
- Budget for data and systems experts at each site (… and then double it)
- Carefully consider bias and take steps to promote equity
- Develop a robust data quality assessment plan to improve value of data and to detect and address data issues – early in data collection

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Concluding points

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

Resource: The Living Textbook

Visit the Living Textbook of Pragmatic Clinical Trials at

www.rethinkingclinicaltrials.org

Pragmatic Trials Collaboratory. Pragmatic clinical trials present an opportunity to efficiently generate high-quality evidence to inform medical decision-making. However, these trials pose different challenges than traditional clinical trials. The Living Textbook reflects a collection of special considerations and best practices in the design, conduct, and reporting of pragmatic clinical trials.

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Question & Answer

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