



Measuring Outcomes

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Essentials of Embedded Pragmatic Clinical Trials

Learning goal

- Describe methods for measuring outcomes using data sources such as electronic health records (EHRs) and patient-reported outcomes (PROs)



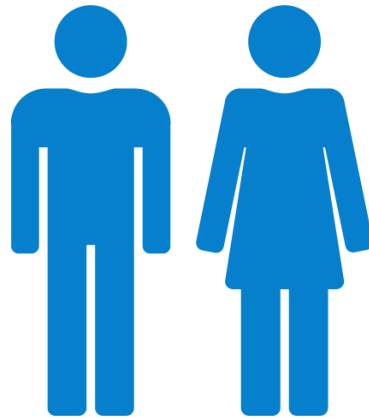
Important things to know

- 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

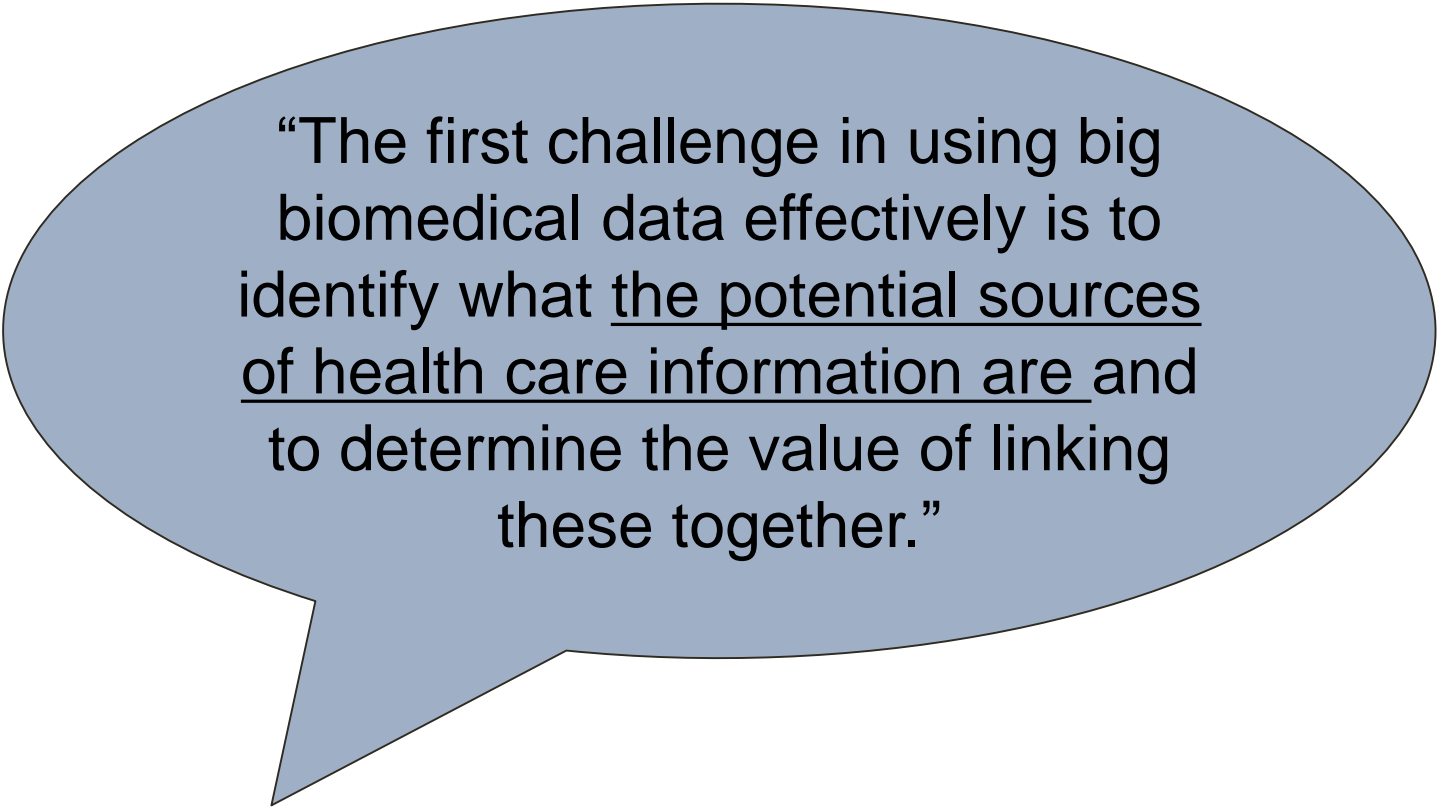
Endpoints and outcomes

An **endpoint** usually refers to an analyzed parameter (eg, change from baseline at 6 weeks in mean PROMIS Fatigue score)

An **outcome** usually refers to a measured variable (eg, peak volume of oxygen or PROMIS Fatigue score)



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.”

Finding the Missing Link for Big Biomedical Data

Griffin M. Weber, MD; Kenneth D. Mandl, MD, MPH; Isaac S. Kohane, MD, PhD.
JAMA. 2014;311(24):2479-2480. doi:10.1001/jama.2014.4228 (*Figure 1*)

Data sources for endpoints in ePCTs

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

Endpoints in ePCTs

- All research endpoints should be meaningful to providers and patients
- 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!

Choosing and specifying endpoints in ePCTs

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



- Acute MI
- Broken bone
- Hospitalization



- 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?

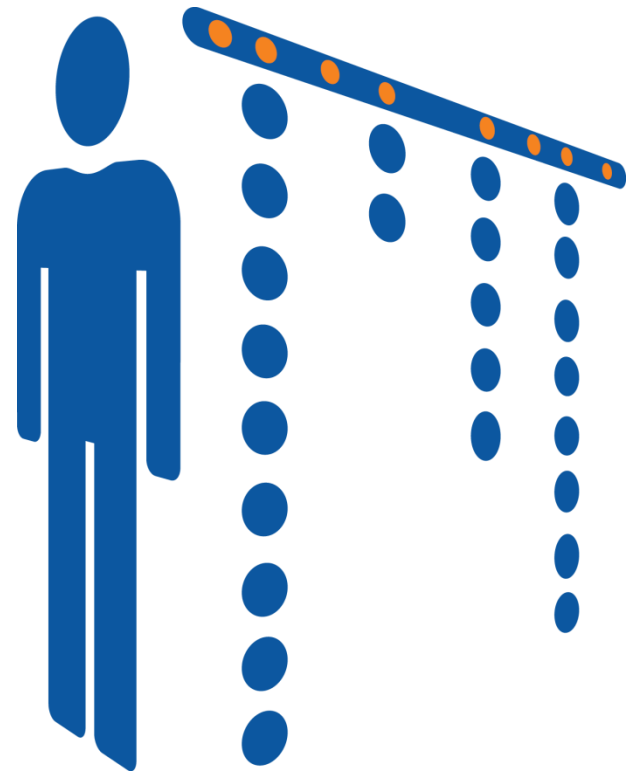
Does it require hospitalization?

Will the endpoint be medically attended?

Is the treatment generally provided in inpatient or outpatient settings?

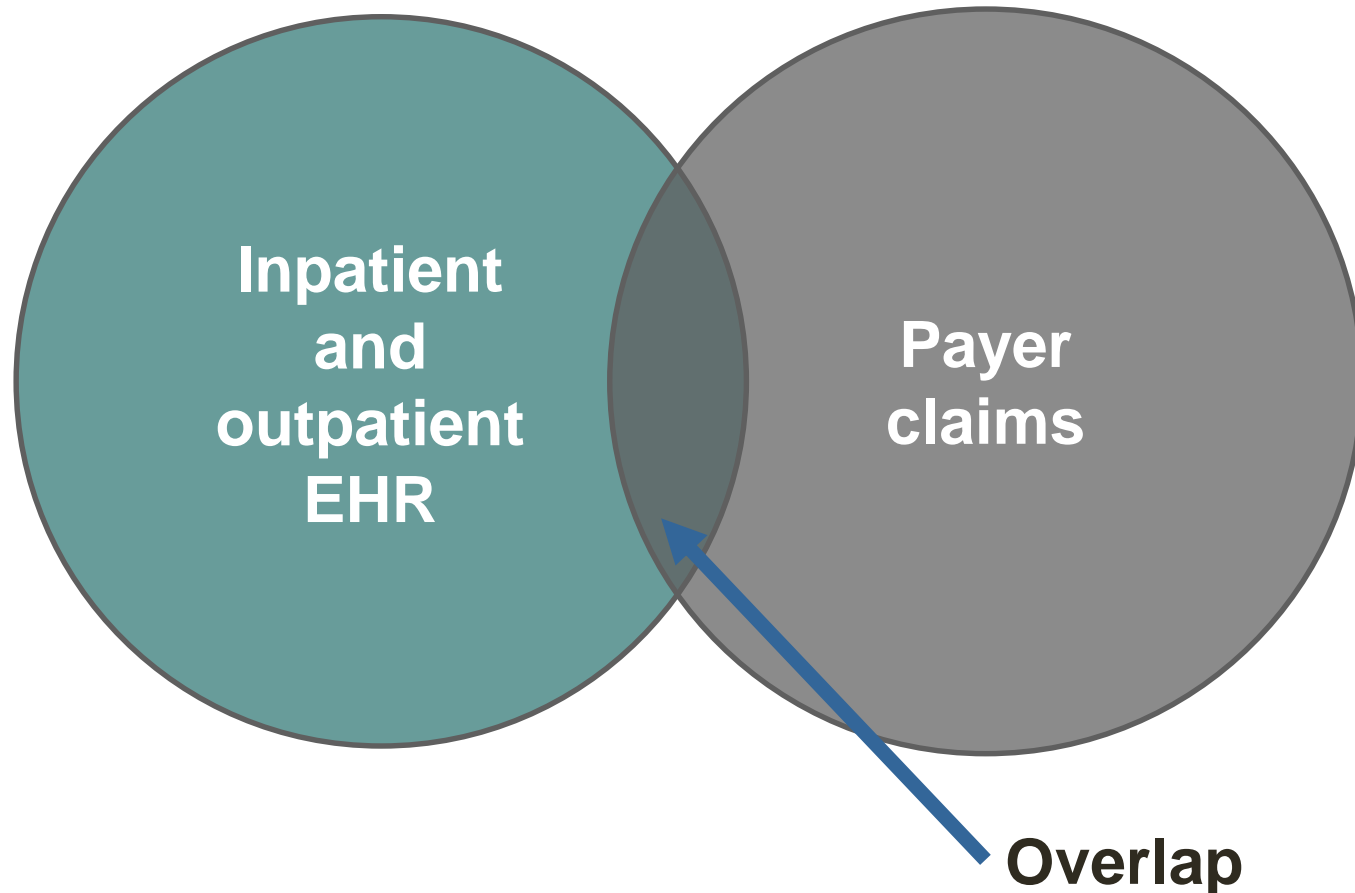
Caveat when using EHR data for research

EHRs often do not tell a complete story

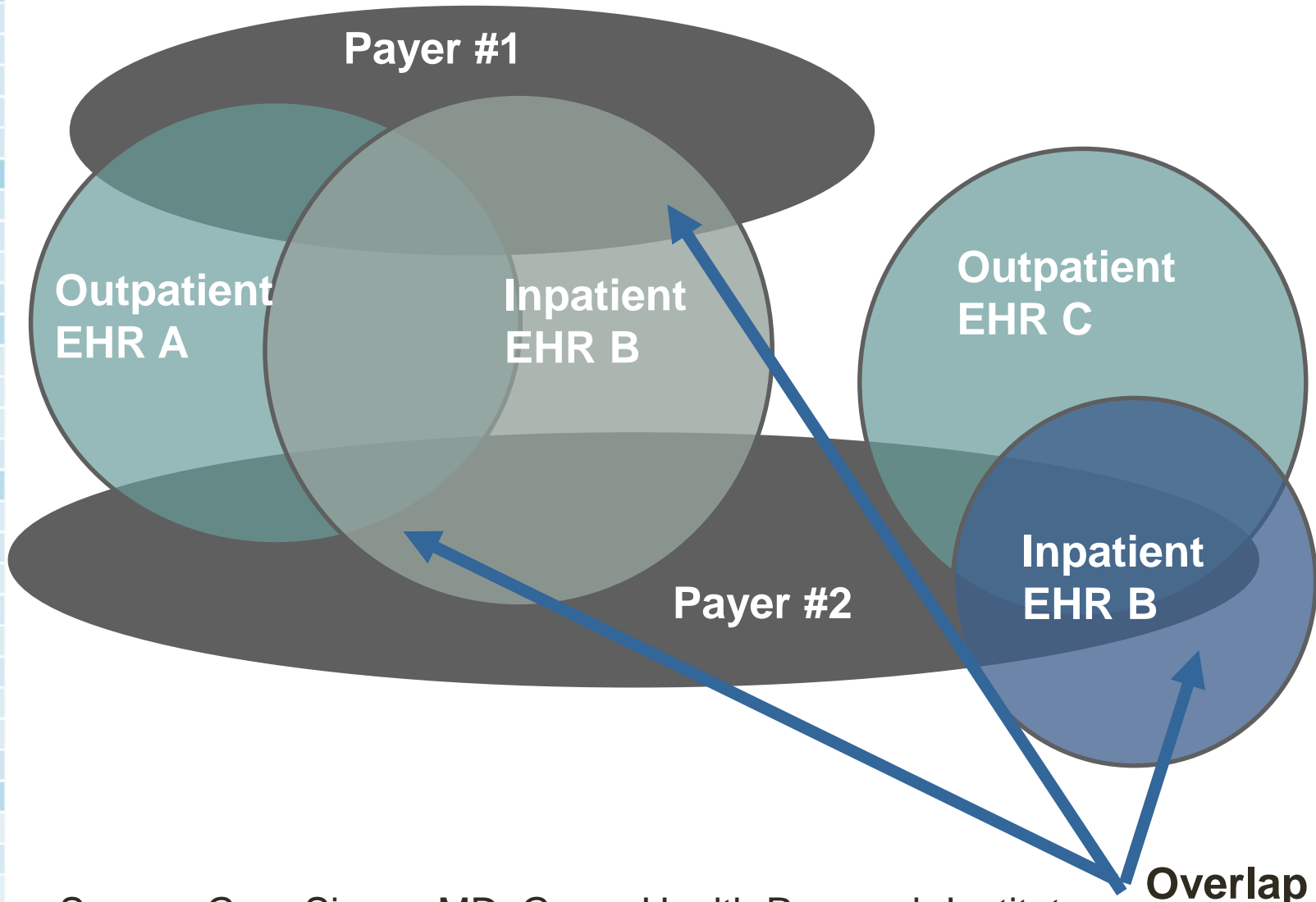


Where is the signal?

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



Reality is not straightforward

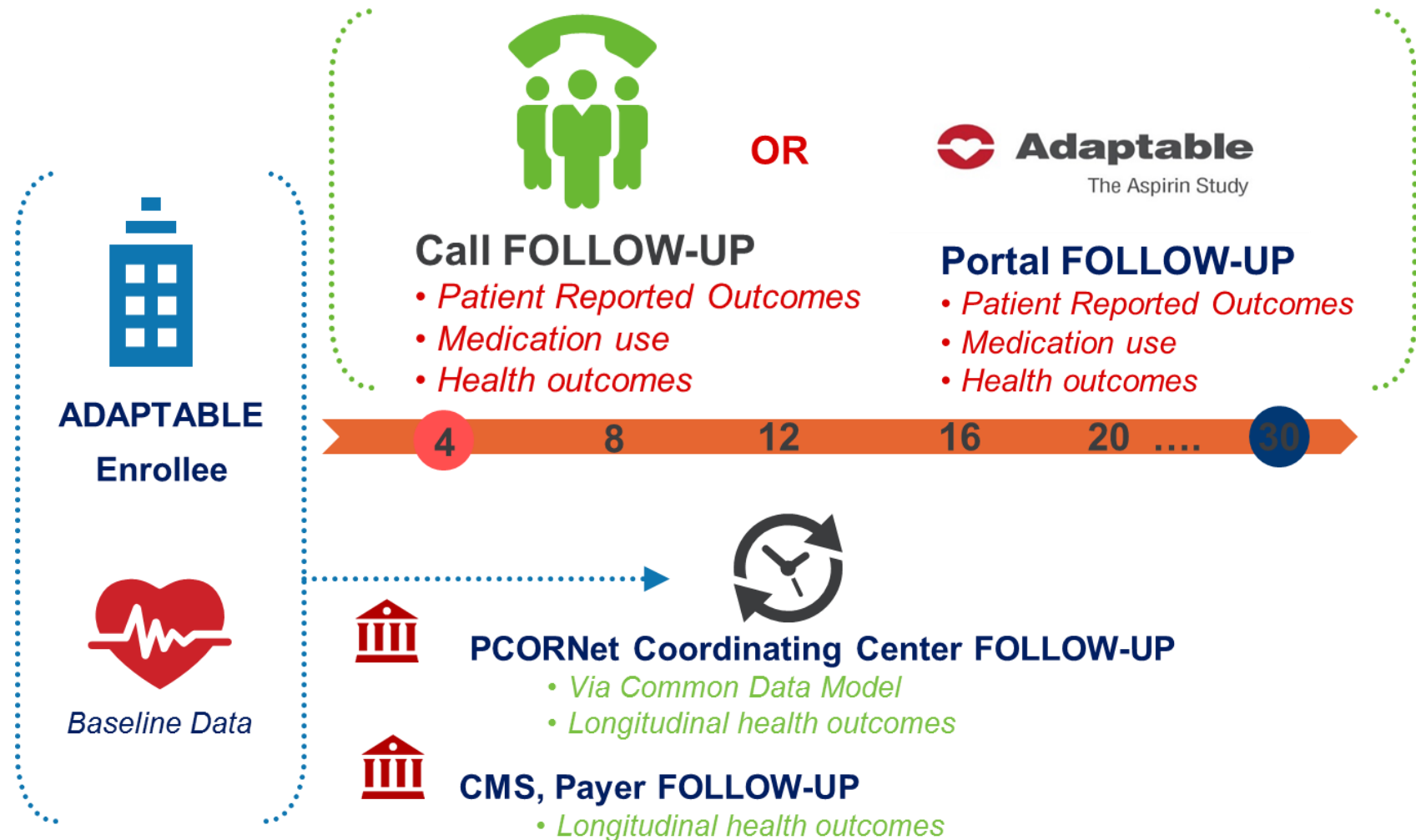


Source: Greg Simon, MD, Group Health Research Institute

Longitudinal data linkage

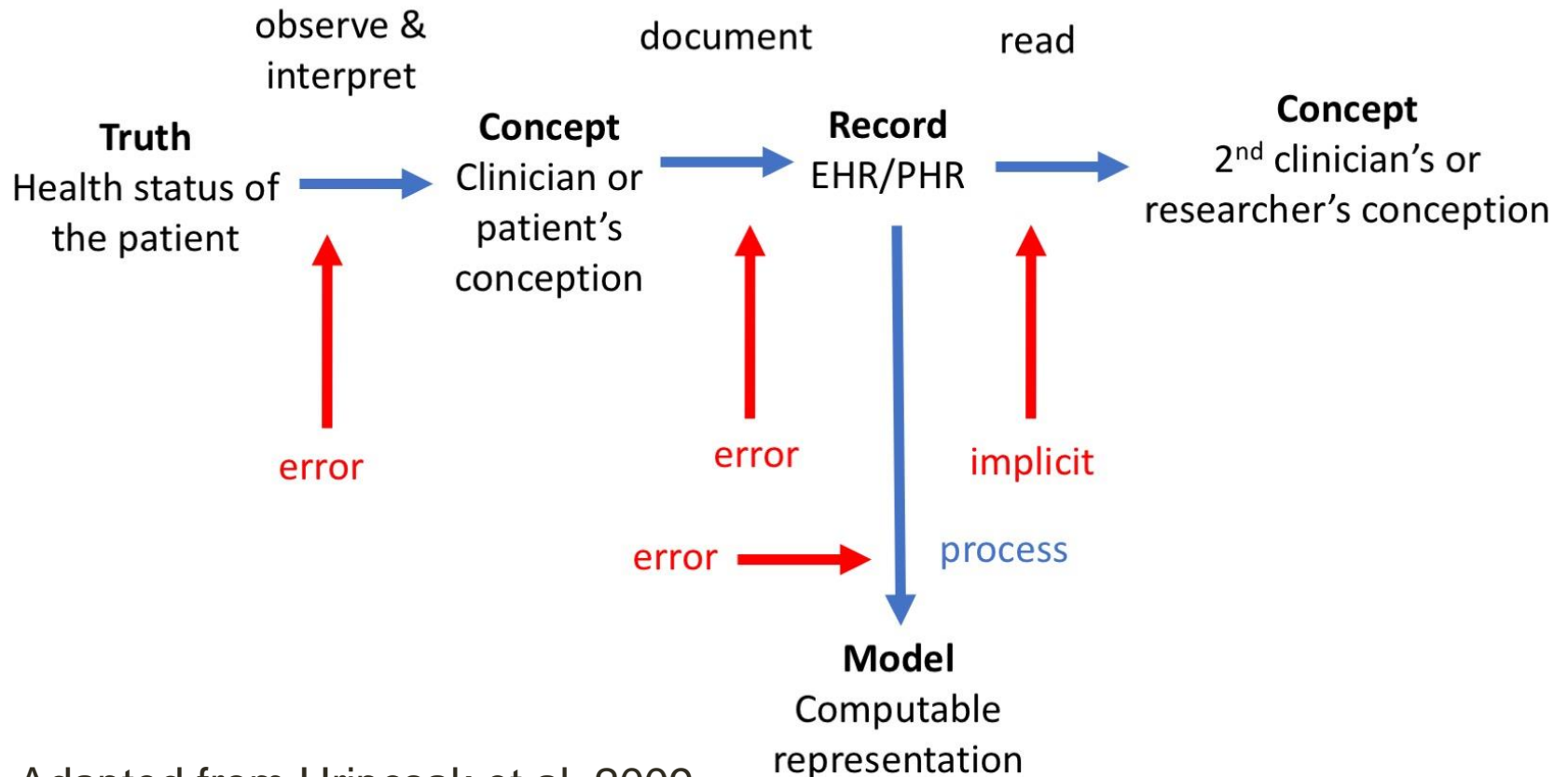
- To fully capture *all* care—complete longitudinal data—linking research & insurance claims data is often necessary
- Without explicit consent, getting longitudinal data from an insurance carrier can be an insurmountable hurdle, both technically and legally

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



Data is a surrogate for clinical phenomena

Error Impact on Trials



Adapted from Hripcsak et al. 2009

Data quality assessment

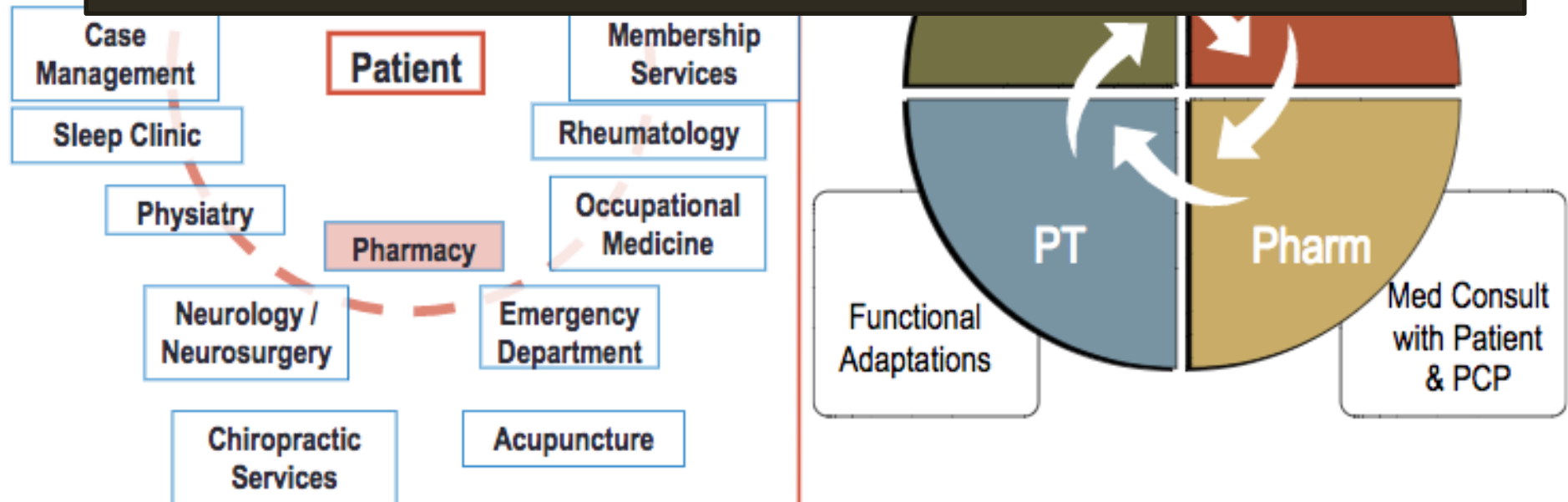
- Identify variation between populations at different sites or study groups
- Recommend formal assessment of accuracy, completeness and consistency for key data
- Data quality should be described, reported and informed by workflows

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

Pain Management: Usual Care

Interdisciplinary Management Embedded in Primary Care

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) 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

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 ePCTs for passive or active surveillance



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



Concluding points

- The data available from the EHR may be convenient and 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



Resource: The Living Textbook

The screenshot shows the homepage of the NIH Collaboratory Living Textbook. The header is dark blue with the text 'NIH COLLABORATORY LIVING TEXTBOOK of Pragmatic Clinical Trials'. Below the header is a navigation bar with links for 'ABOUT', 'RESOURCES', 'GRAND ROUNDS', and 'NEWS', along with a search bar. The main content area is divided into three columns: 'DESIGN' (purple background), 'DATA, TOOLS & CONDUCT' (dark blue background), and 'DISSEMINATION' (green background). Each column lists specific topics related to its category.

DESIGN	DATA, TOOLS & CONDUCT	DISSEMINATION
Developing a Compelling Grant Application Experimental Designs and Randomization Schemes Endpoints and Outcomes	Analysis Plan Consent, Disclosure, and Non-disclosure Using Electronic Health Record Data	Data and Safety Monitoring Designing With Implementation and Dissemination in Mind Embedded PCT Team Composition

Choosing and Specifying Endpoints and Outcomes

- [Introduction](#)
- [Meaningful Endpoints](#)
- [Outcomes Measured via the Electronic Health Record](#)
- [Using Death as an Endpoint](#)
- [Inpatient Endpoints in Pragmatic Clinical Trials](#)
- [Outcomes Measured via Direct Patient Report](#)
- [Outcomes Measured via Mobile Devices](#)
- [Additional Resources](#)
- [FAQ](#)