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)

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)

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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 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

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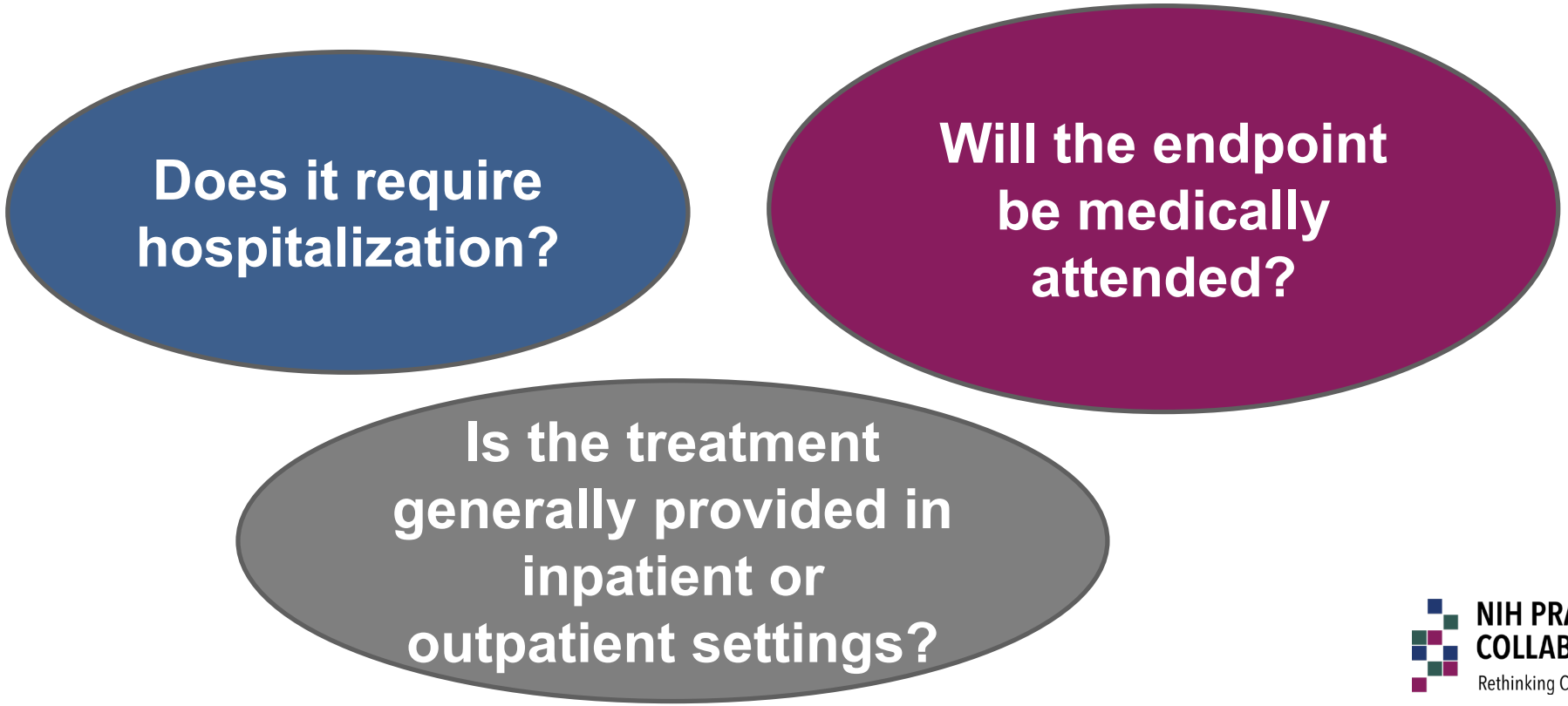
Key questions for choosing endpoints

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



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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.

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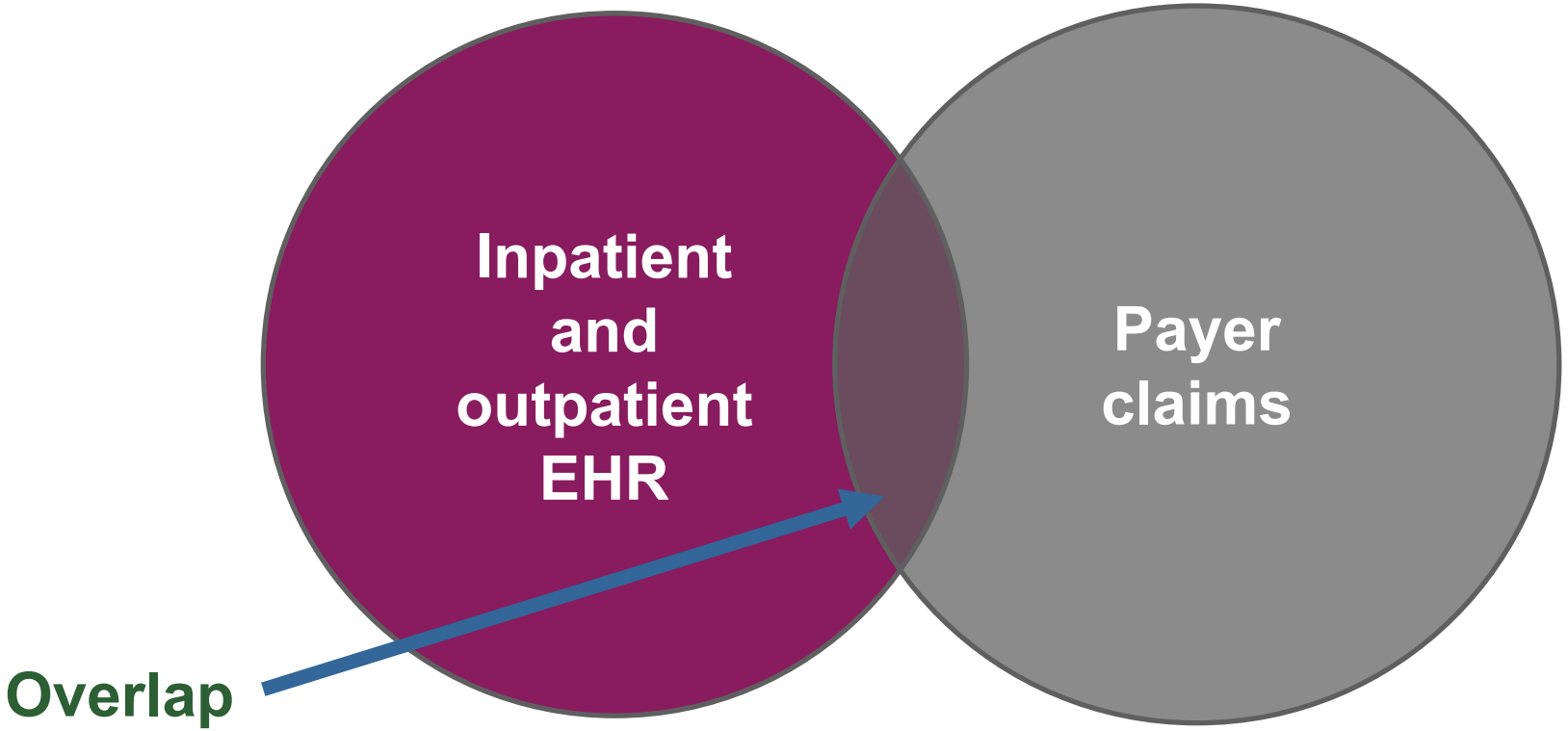
Where is the signal?

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



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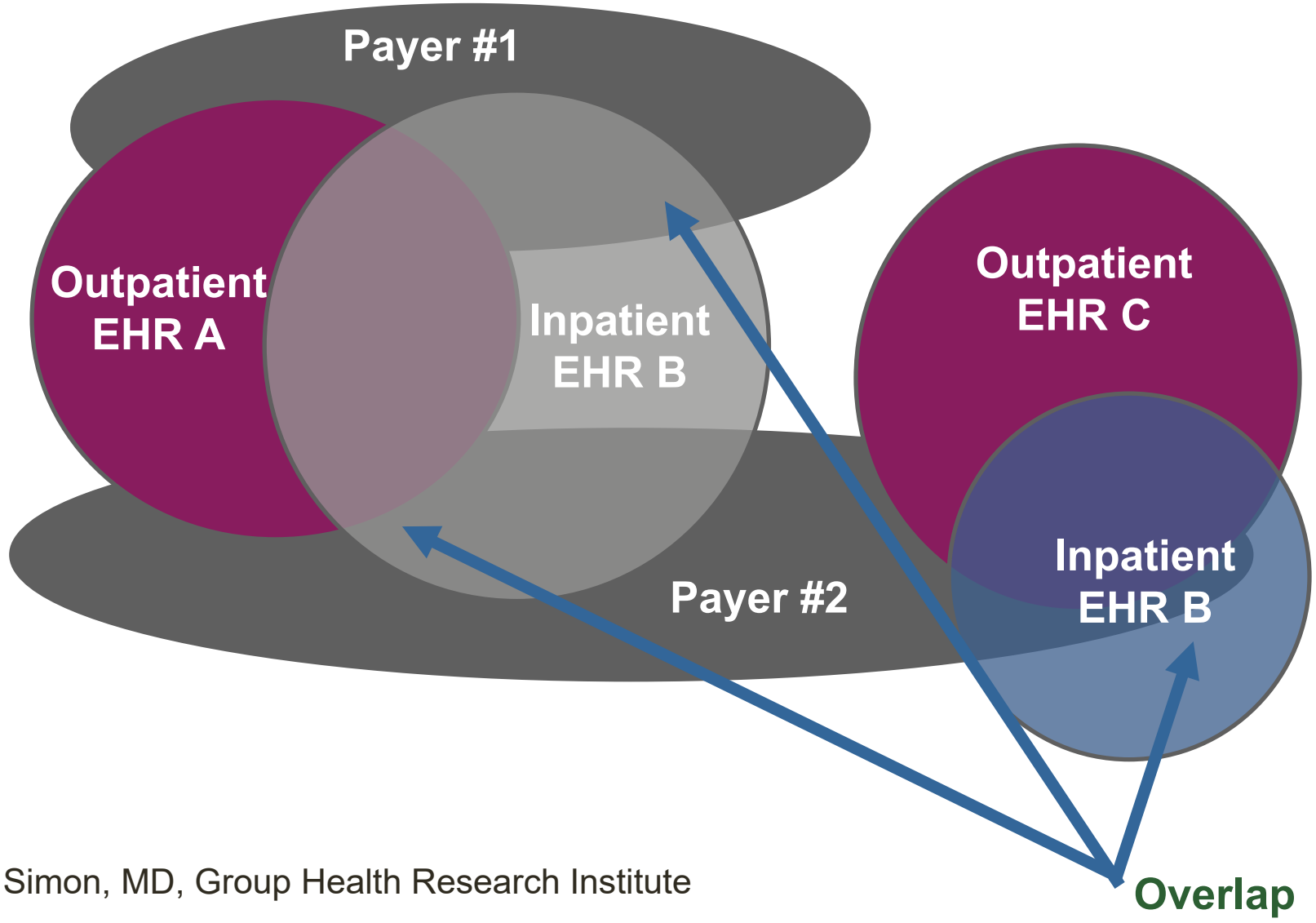
Reality is not straightforward



Source: Greg Simon, MD, Group Health Research Institute

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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

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

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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

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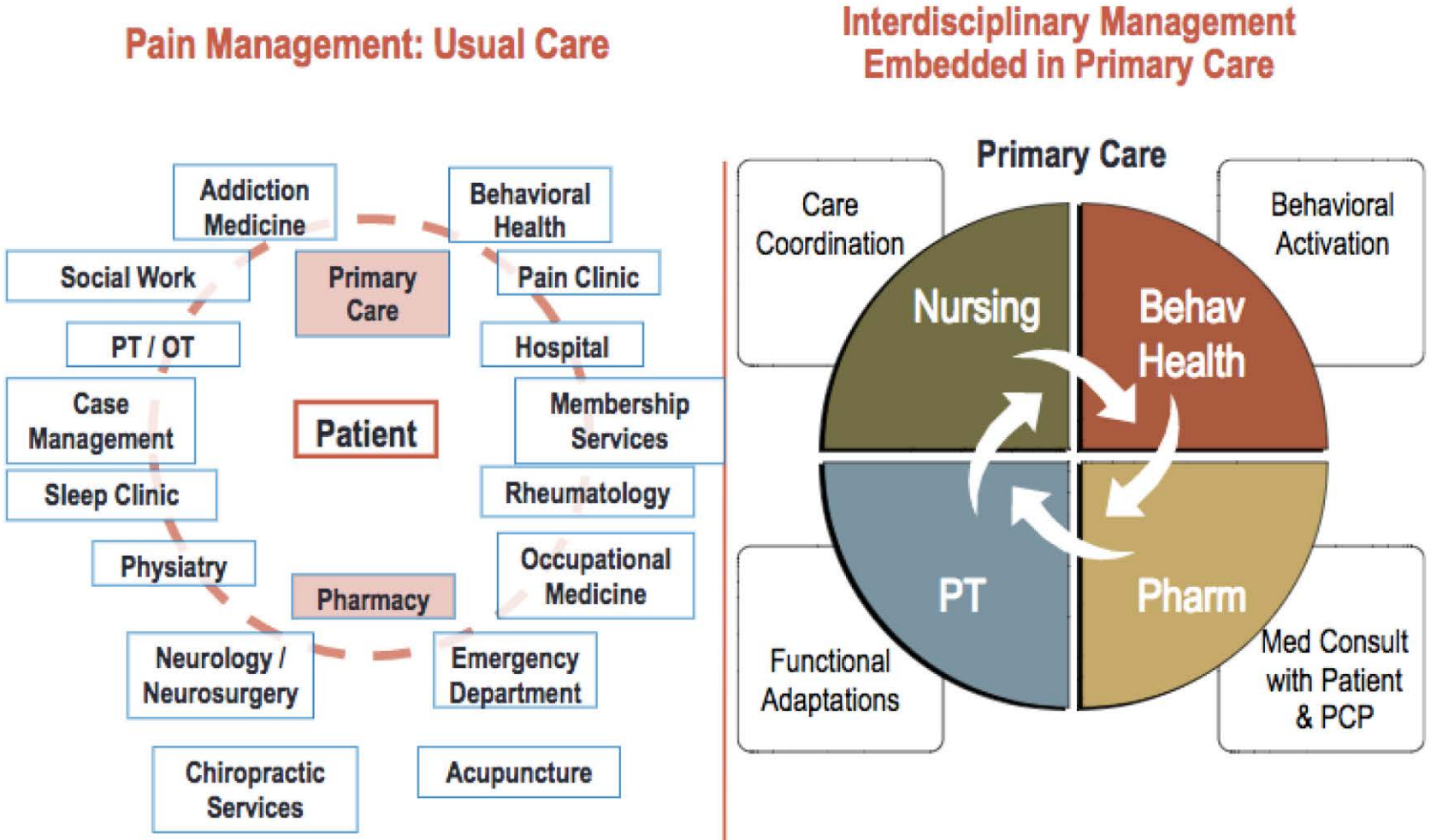
Case example: Collaborative Care for Chronic Pain in Primary Care (PPACT)



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

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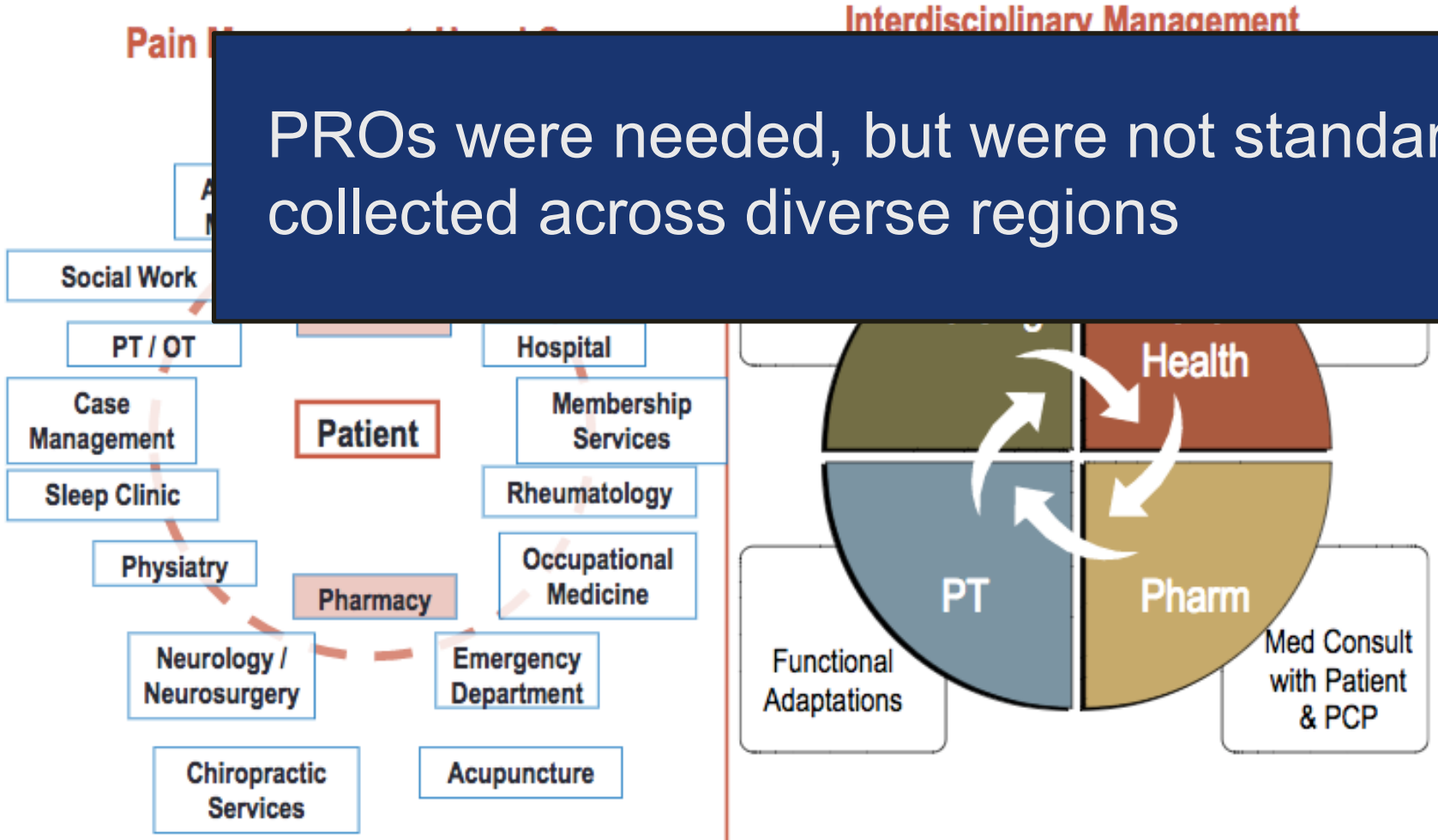
Case example: Collaborative Care for Chronic Pain in Primary Care (PPACT)

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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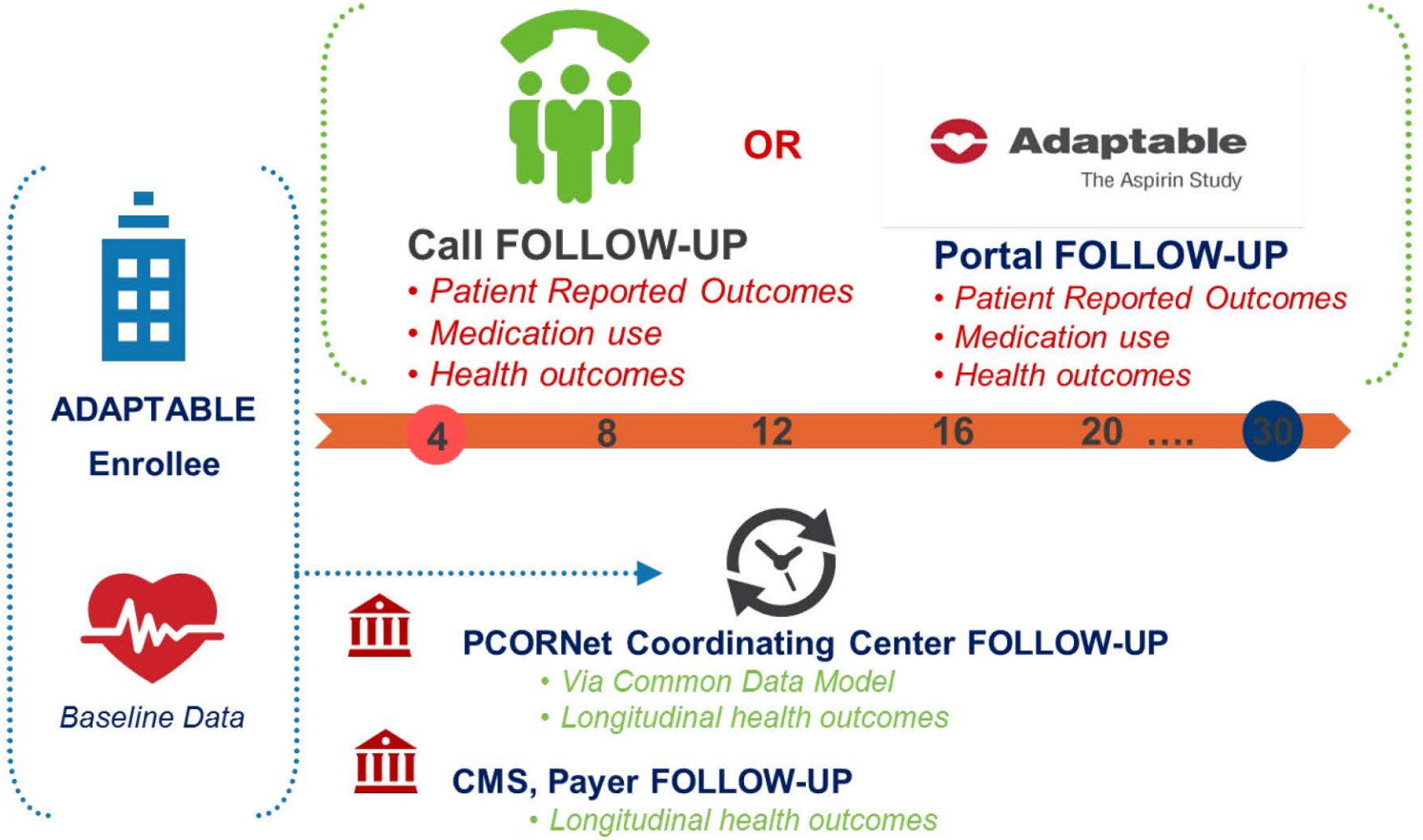
Case example: PPACT

- Project leadership worked with national Kaiser to create buy-in for a common instrument
- Local IT built it within each region
- A 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

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



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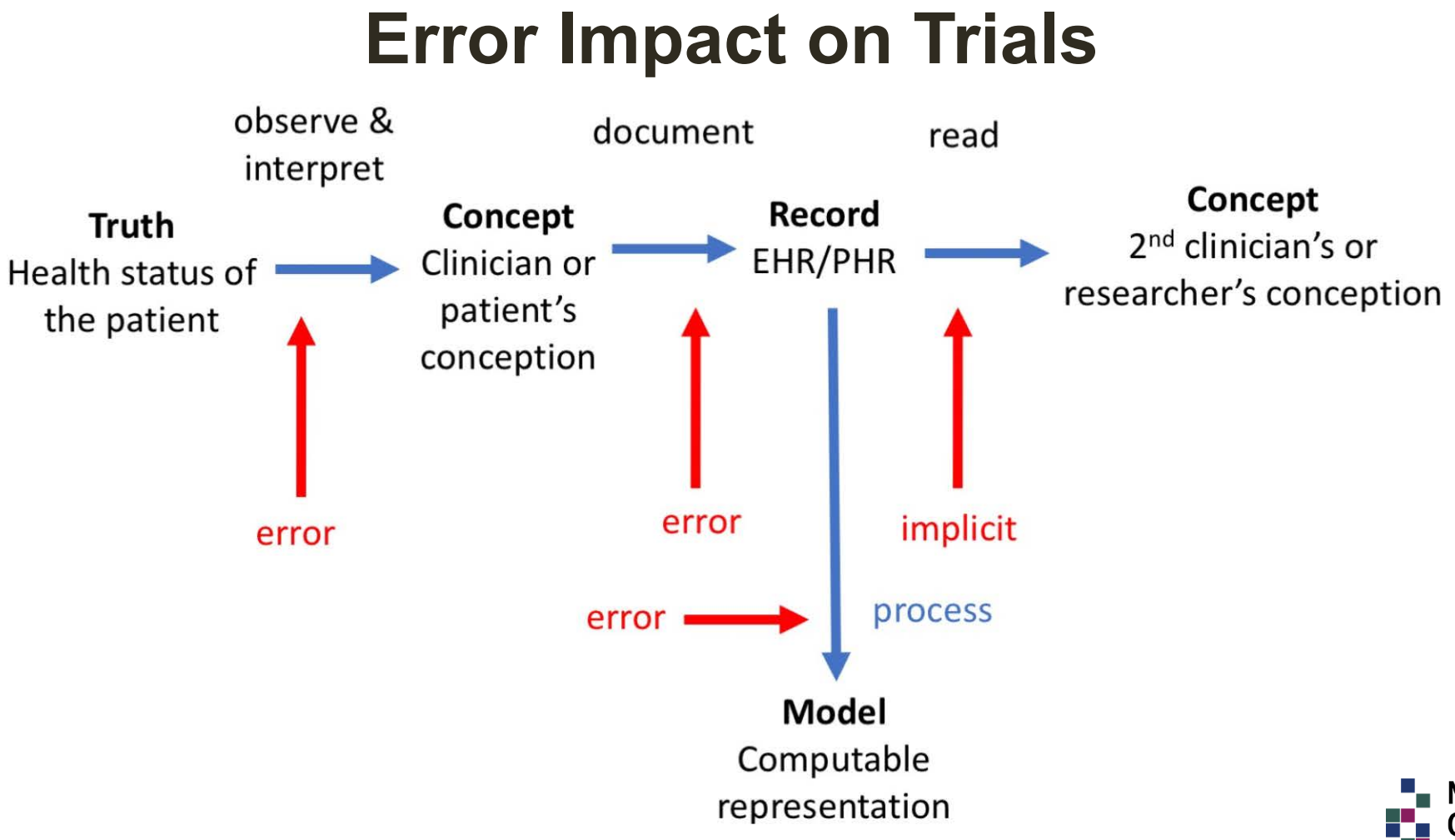
Mobile devices for outcome measurement

- 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



Adapted from Hripcsak et al. 2009

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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 not actually drive clinical practice or policy if used as endpoints
- Need to make sure that conveniently available endpoint will also be accepted as influential for stakeholders when the ePCT results are disseminated
- Plan with implementation in mind

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Resource: The Living Textbook

Choosing and Specifying Endpoints and Outcomes

SECTIONS

1. Introduction
2. Meaningful Endpoints
3. Outcomes Measured via the Electronic Health Record
4. Using Death as an Endpoint
5. Inpatient Endpoints in Pragmatic Clinical Trials
6. Outcomes Measured via Direct Patient Report
7. Outcomes Measured via Mobile Devices
8. Additional Resources
9. FAQ

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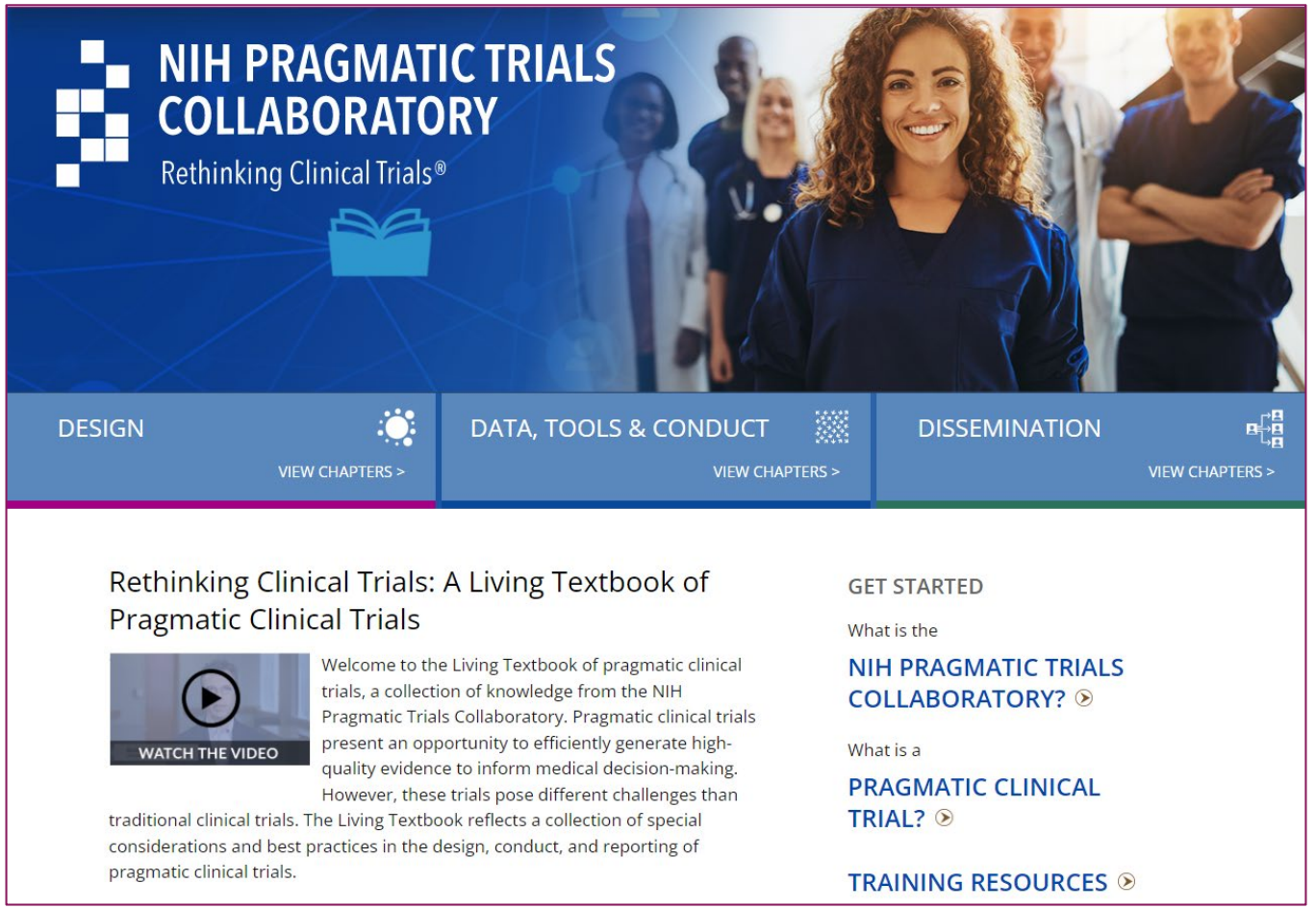
Resource: The Living Textbook

Visit the Living Textbook of Pragmatic Clinical Trials at

www.rethinkingclinicaltrials.org



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