Innovative Methods in ePCTs

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Disclosures

- Dr. Angelo Volandes has a financial interest in ACP Decisions, a nonprofit organization developing advance care planning video decision support tools. Dr. Volandes' interests were reviewed and are managed by Dartmouth in accordance with their conflict-of-interest policies. No other disclosures to report.

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Learning goals

- Describe methods for measuring outcomes using real-world data sources such as electronic health records (EHRs) and patient-reported outcomes (PROs)
- Discuss how the latest information capabilities, such as AI, can enhance ePCTs
- Identify considerations for the use of new data technologies in ePCTs, including ethical issues

Outcome, measure, endpoint

- **Outcome** usually refers to a variable of interest or a meaningful aspect of health (such as oxygen volume or fatigue)

- **Measure** usually refers to a specific, standardized process to obtain information on an outcome
  - Includes instructions, administration materials, content, formatting, and scoring rules

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Types of measures

Patient-reported outcome measure (PROM)

Observer-reported outcome measure (ObsRO)

Clinician-reported outcome measure (ClinRO)

Performance outcome measure (PerfO)

Outcome, measure, endpoint

- **Endpoint** usually refers to a precisely defined variable that is statistically analyzed to address a particular research question

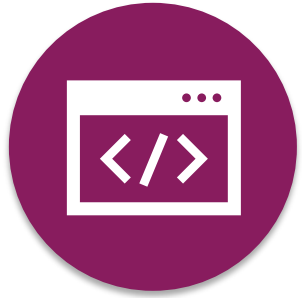
Examples:

- Change from baseline at 6 weeks in mean PROMIS Fatigue score
- Mean difference in PROMIS Fatigue score between patients in the intervention and usual 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 (ie, pragmatic)



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

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Choosing and specifying endpoints in ePCTs

Outcomes and related endpoints should be available as part of routine care

- Acute myocardial infarction
- Broken bone
- Hospitalization

- Suicide attempts
- Gout flares
- Silent myocardial infarction
- Early miscarriage

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Key questions for choosing endpoints

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

- Does it require hospitalization?
- Is treatment provided in inpatient or outpatient settings?
- Will the event be medically attended?

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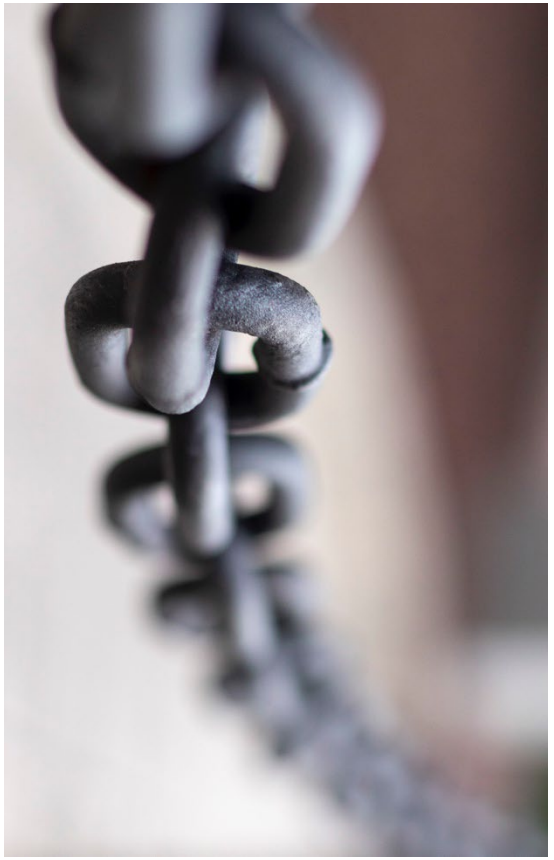
Data sources for endpoints in ePCTs

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



Weber GM, Mandl KD, Kohane IS. Finding the missing link for big biomedical data. *JAMA*. 2014 Jun 25;311(24):2479-80. doi: 10.1001/jama.2014.4228.

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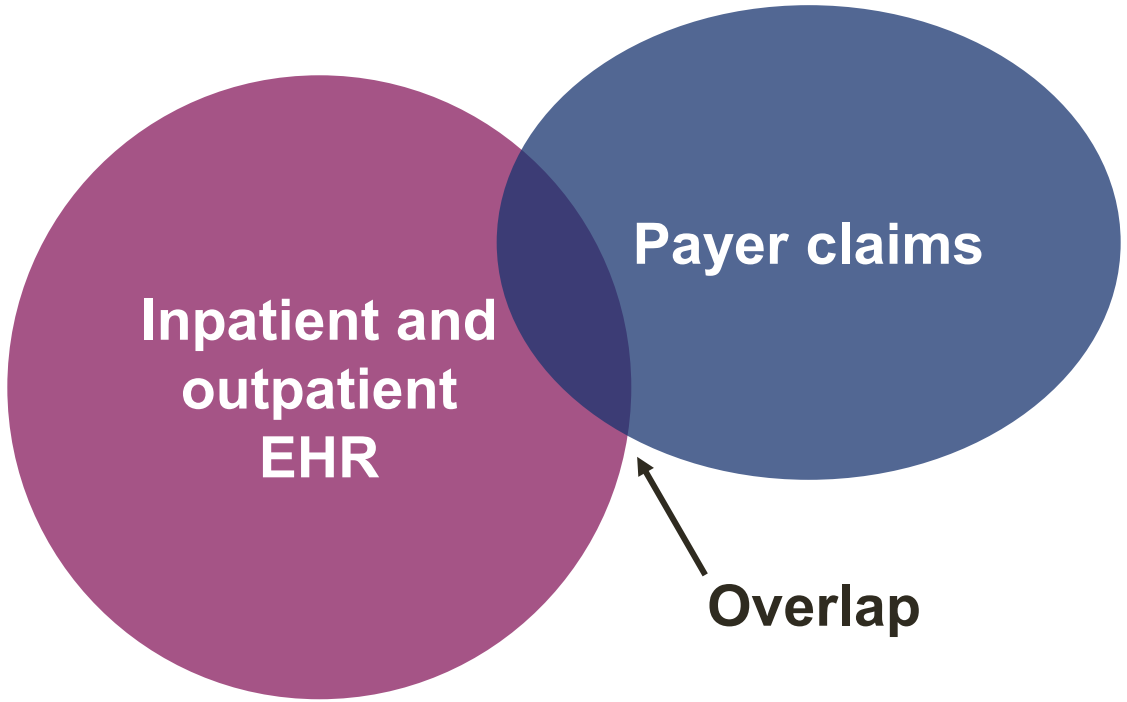
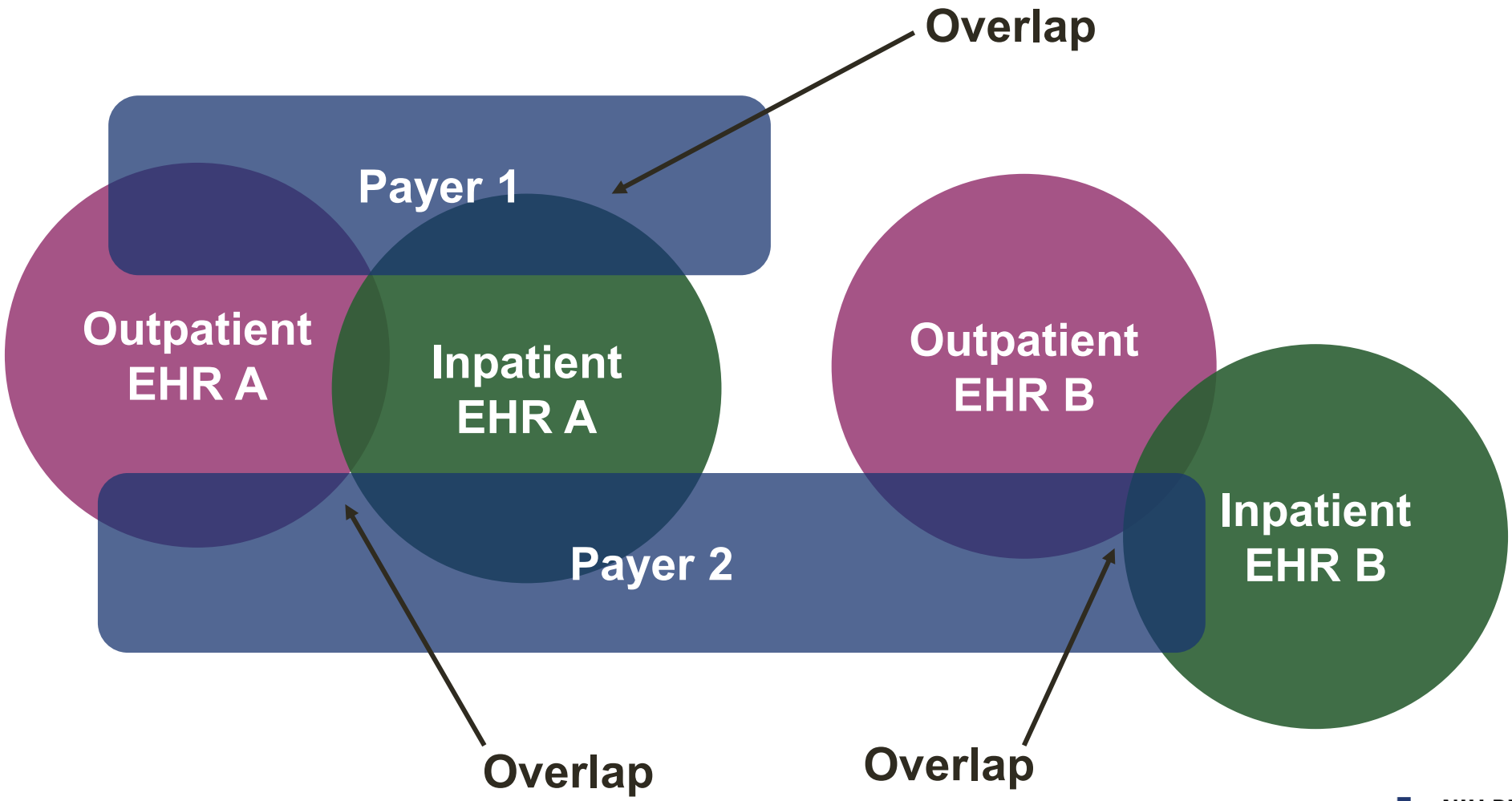
Where is the signal?

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



Reality is not straightforward



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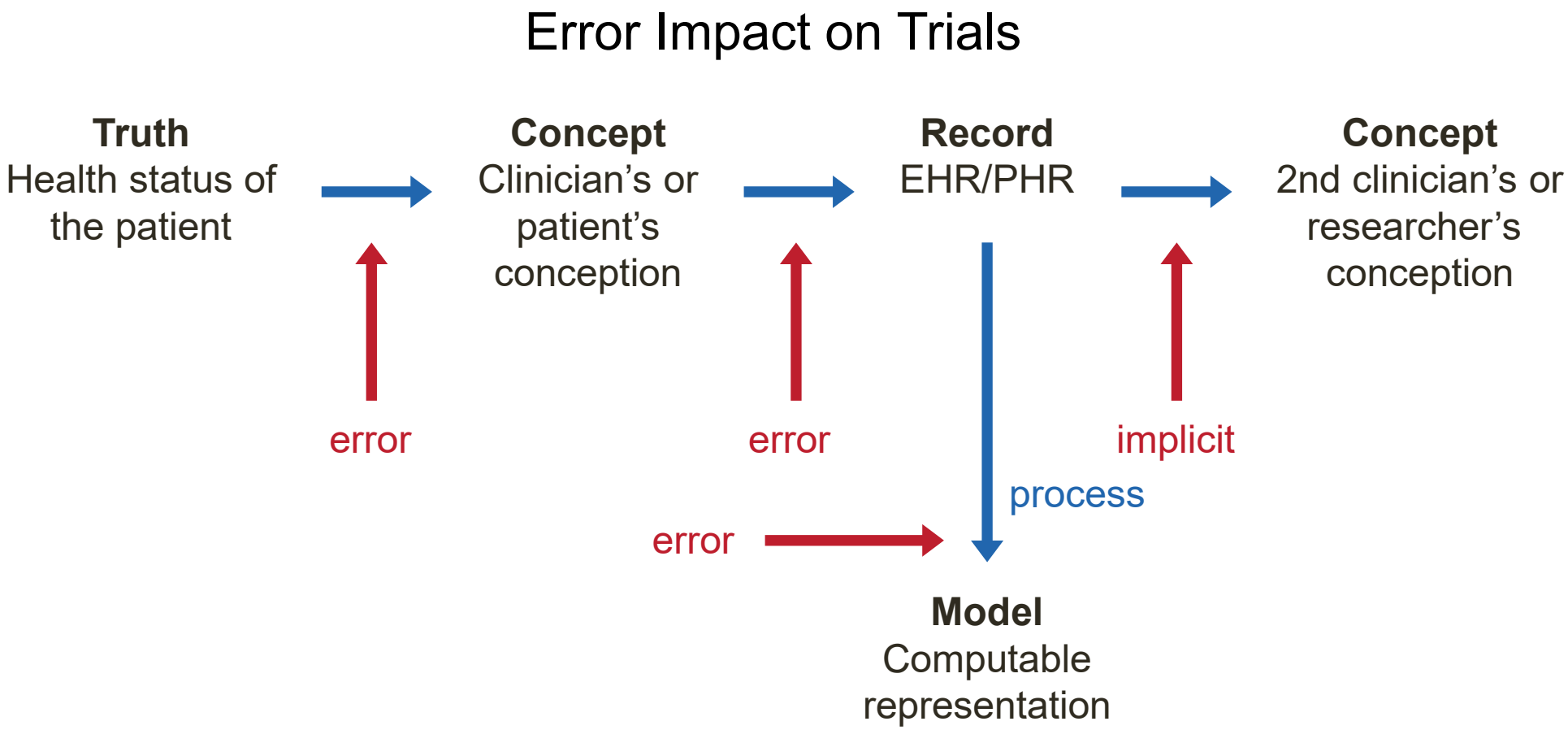
Longitudinal data linkage

- 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



Adapted from Hripcsak et al. J Am Med Inform Assoc. 2009 Mar-Apr;16(2):220-7.

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Data sources for endpoints in ePCTs

Traditional

- EHR or ancillary health information systems

Complementary

- Other types of health data not routinely collected outside standard clinical practice, such as PROs

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It’s a balancing act

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

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

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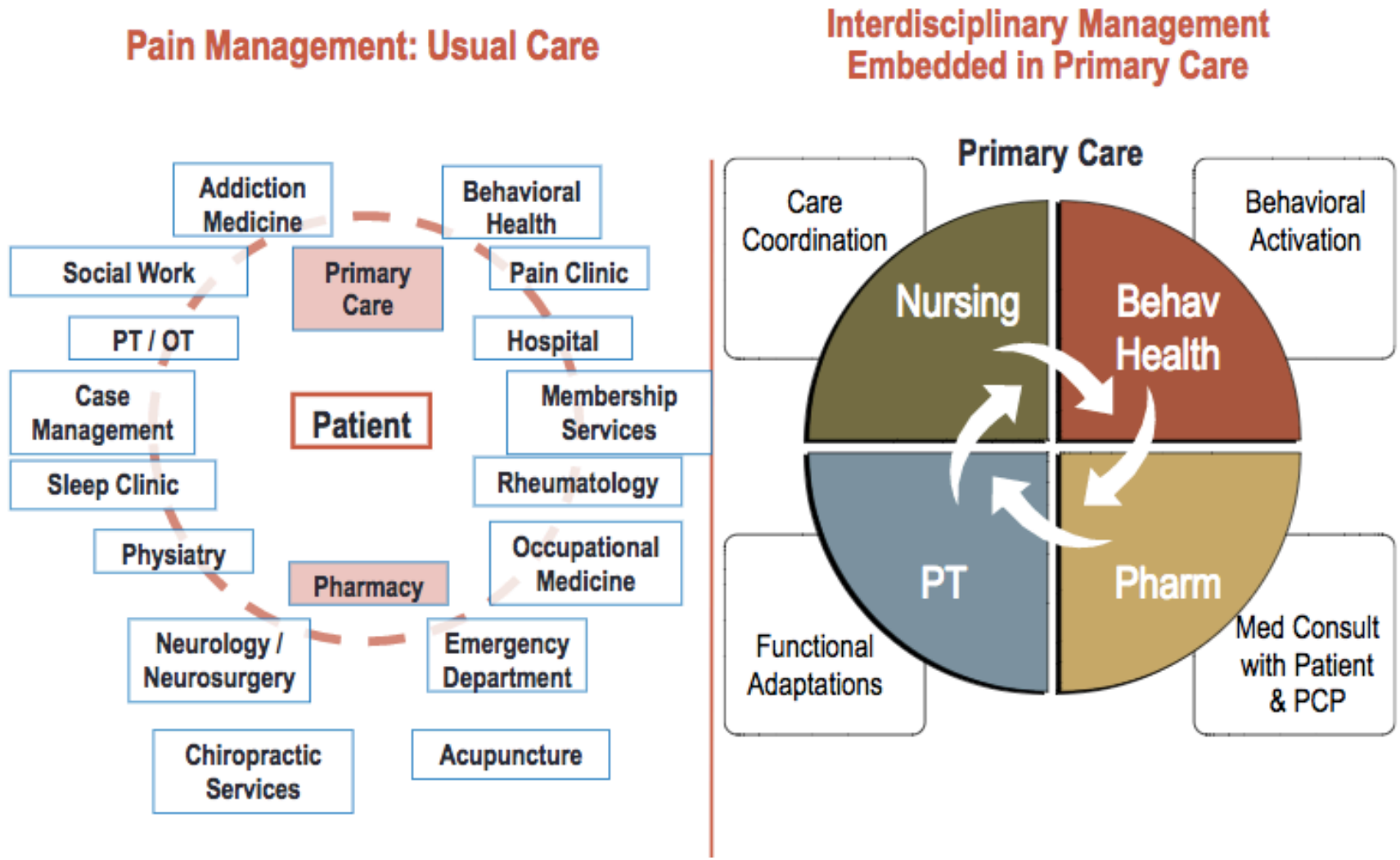
Outcomes measured via direct patient report

- 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

Example: PPACT Trial



Source: Lynn DeBar, Kaiser Permanente Washington Health Research Institute

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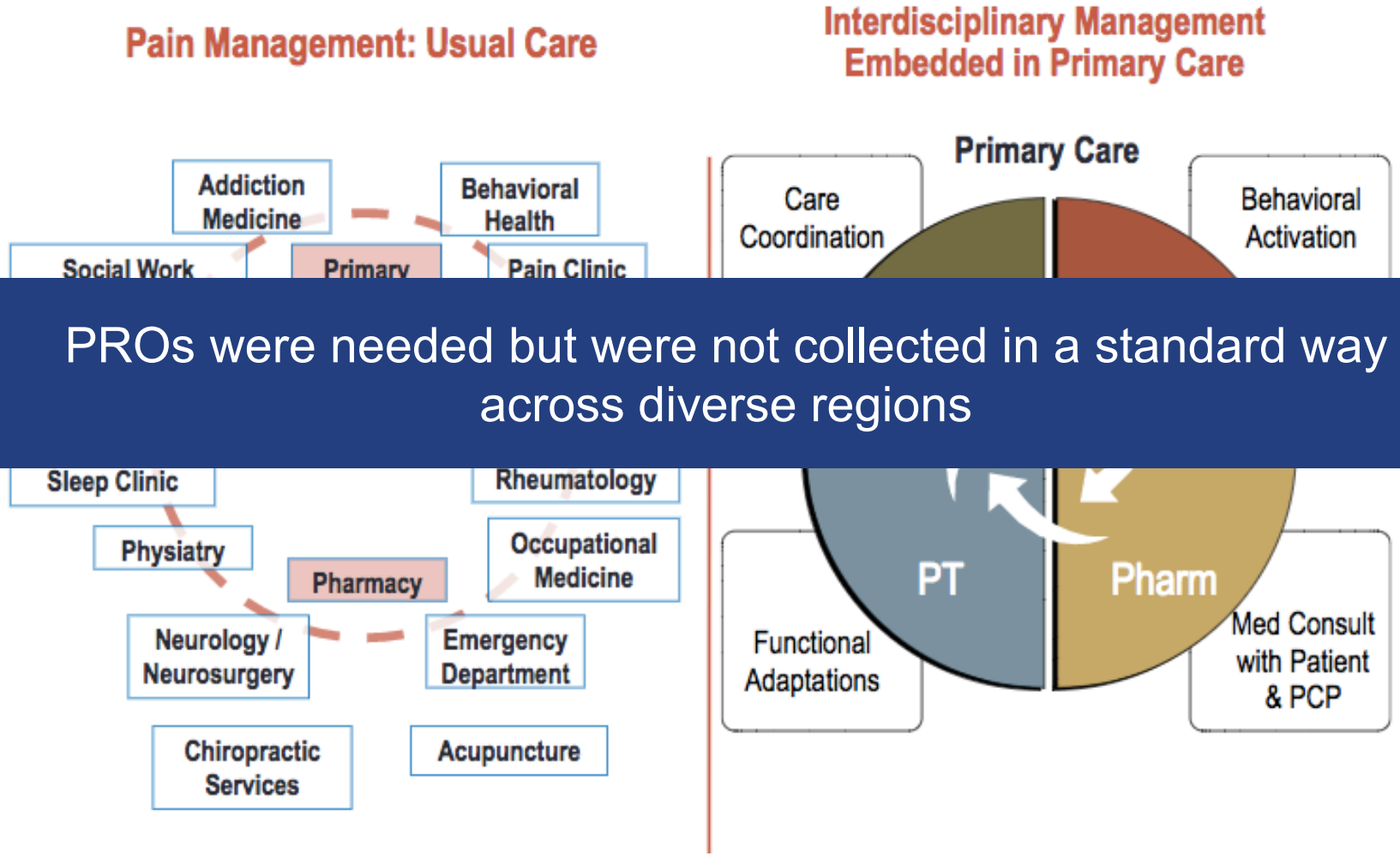
Example: PPACT Trial



PROs were needed but were not collected in a standard way across diverse regions

Source: Lynn DeBar, Kaiser Permanente Washington Health Research Institute

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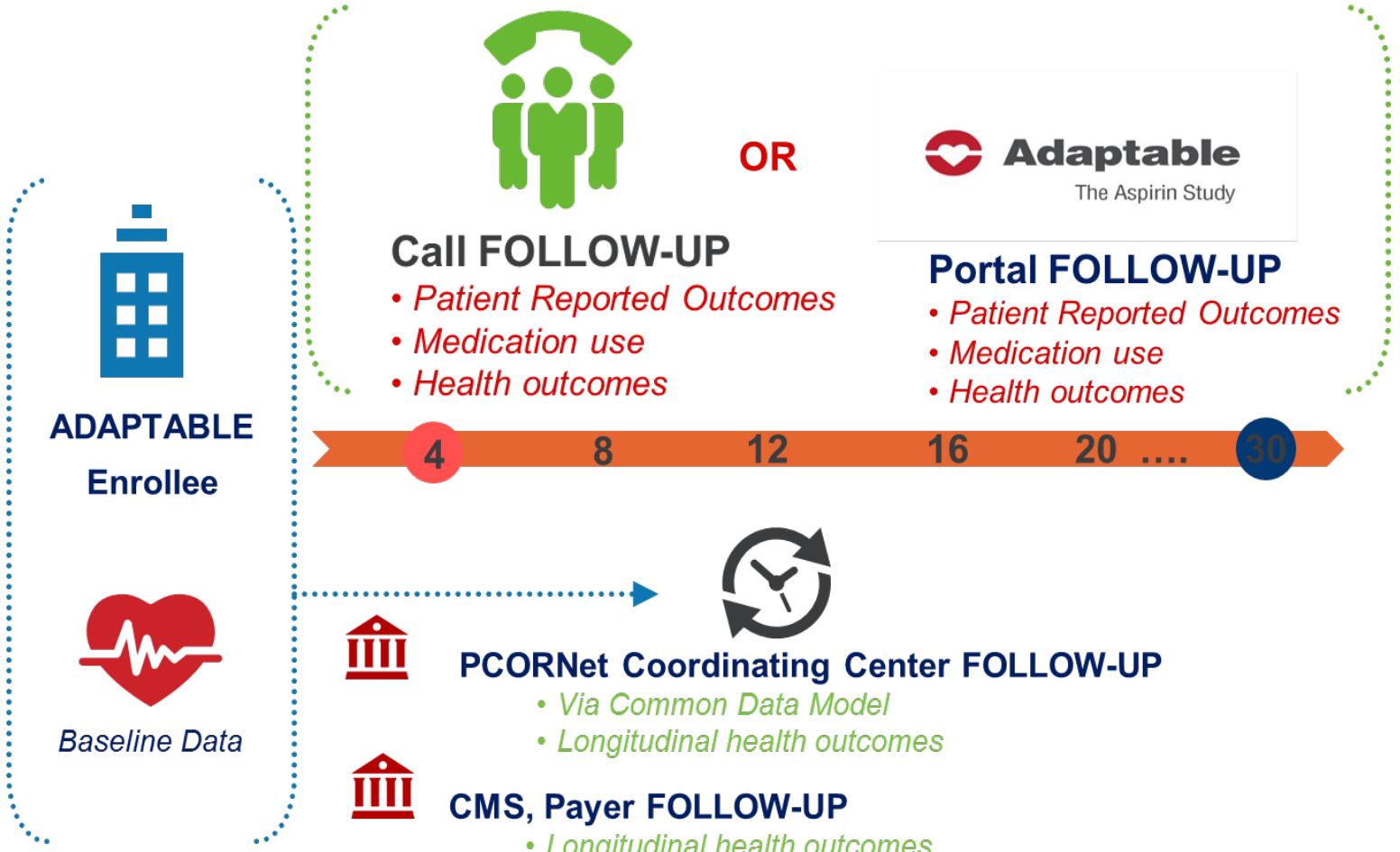
Example: PPACT Trial

- Project leaders worked with national Kaiser Permanente to create buy-in for a common instrument
- Local IT staff built it within each region
- A multitiered approach supplemented PROM data collected in clinics 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

Enabling pragmatic research

E-screening, e-enrollment, and 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

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Data quality assessment

- Identify variation between populations at different sites or in different 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
- 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 (…then double it)
- Carefully consider bias and take steps to promote generalizability
- Develop a data quality assessment plan to improve the value of the data and detect and address data issues early

Q&A

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Knowledge checkpoint

- Getting longitudinal data from an insurance carrier does not require explicit consent from the patient.
  - True
  - False

Knowledge checkpoint

- Suicide attempts would be a good endpoint to use in pragmatic clinical trials since they are routinely documented.
  - True
  - False

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Knowledge checkpoint

- Mobile devices are promising means of obtaining data although presently largely imperfect measures.
  - True
  - False

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