

# Measuring Outcomes

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**NIH PRAGMATIC TRIALS  
COLLABORATORY**

Rethinking Clinical Trials®

# Disclosures

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

# 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



- 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 event be medically attended?

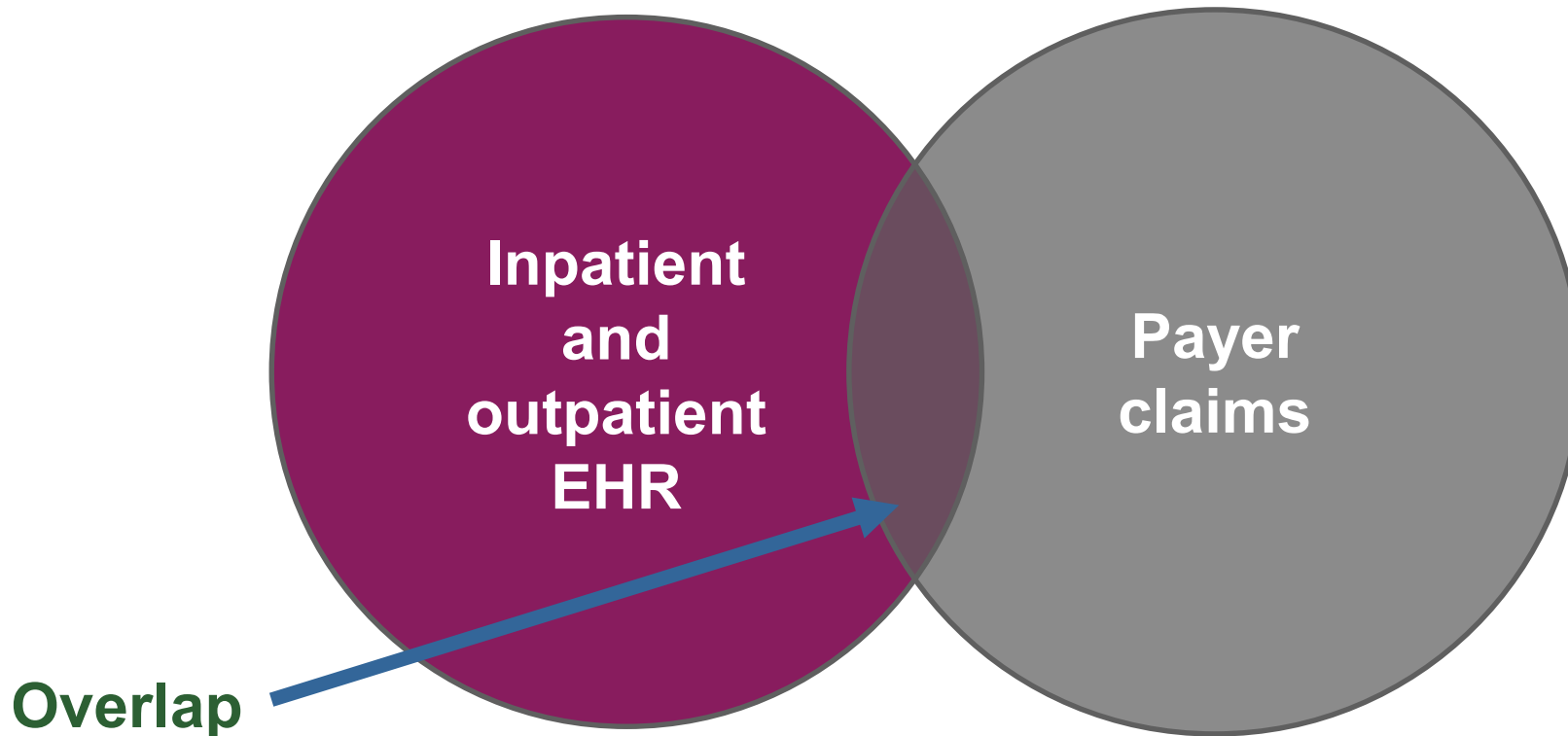
Is the treatment generally provided in inpatient or outpatient settings?

# Data sources for endpoints in ePCTs

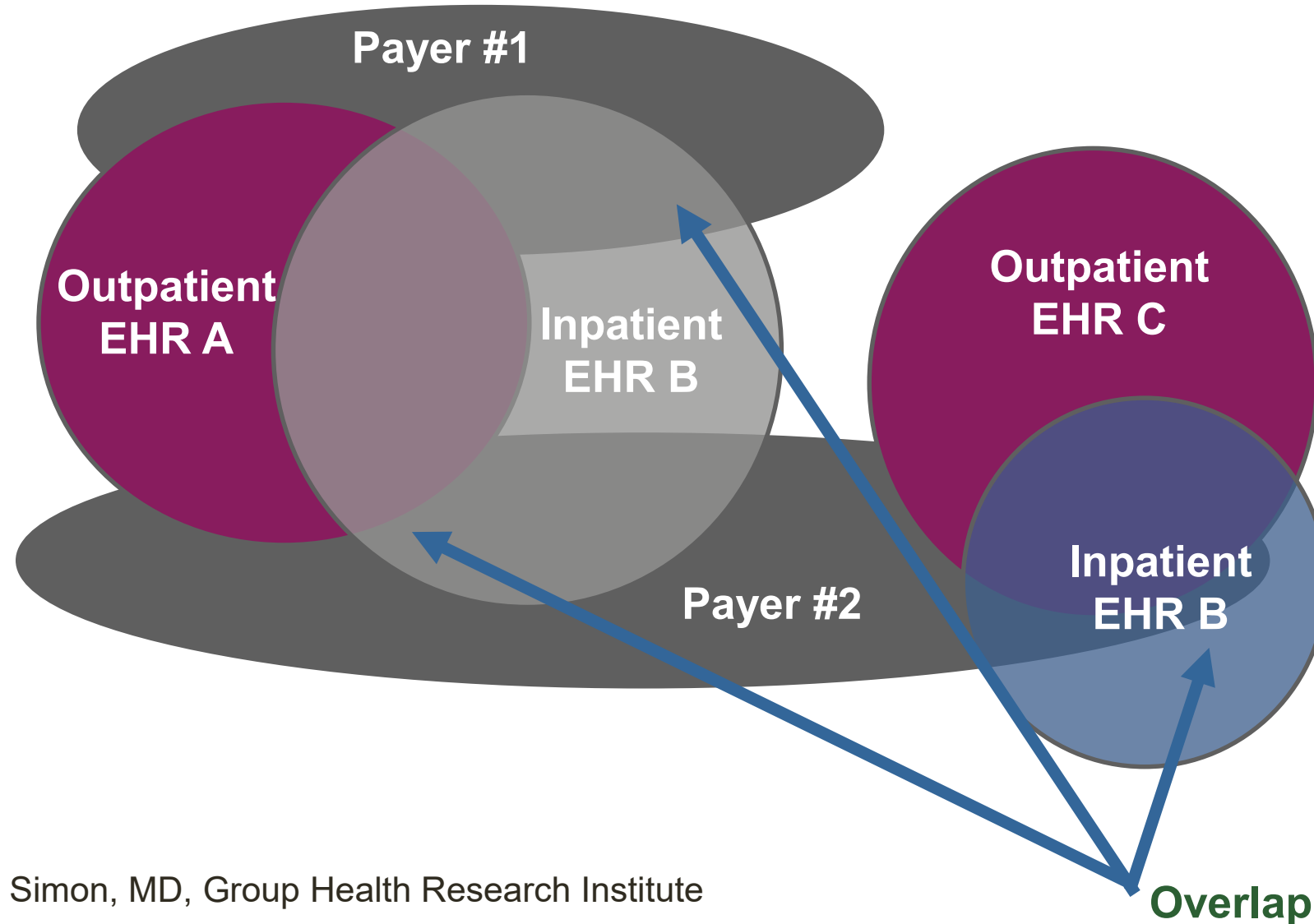
“The first challenge in using big biomedical data effectively is to identify what the potential sources of health care information are and to determine the value of linking these together.”

# Where is the signal?

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



# Reality is not straightforward



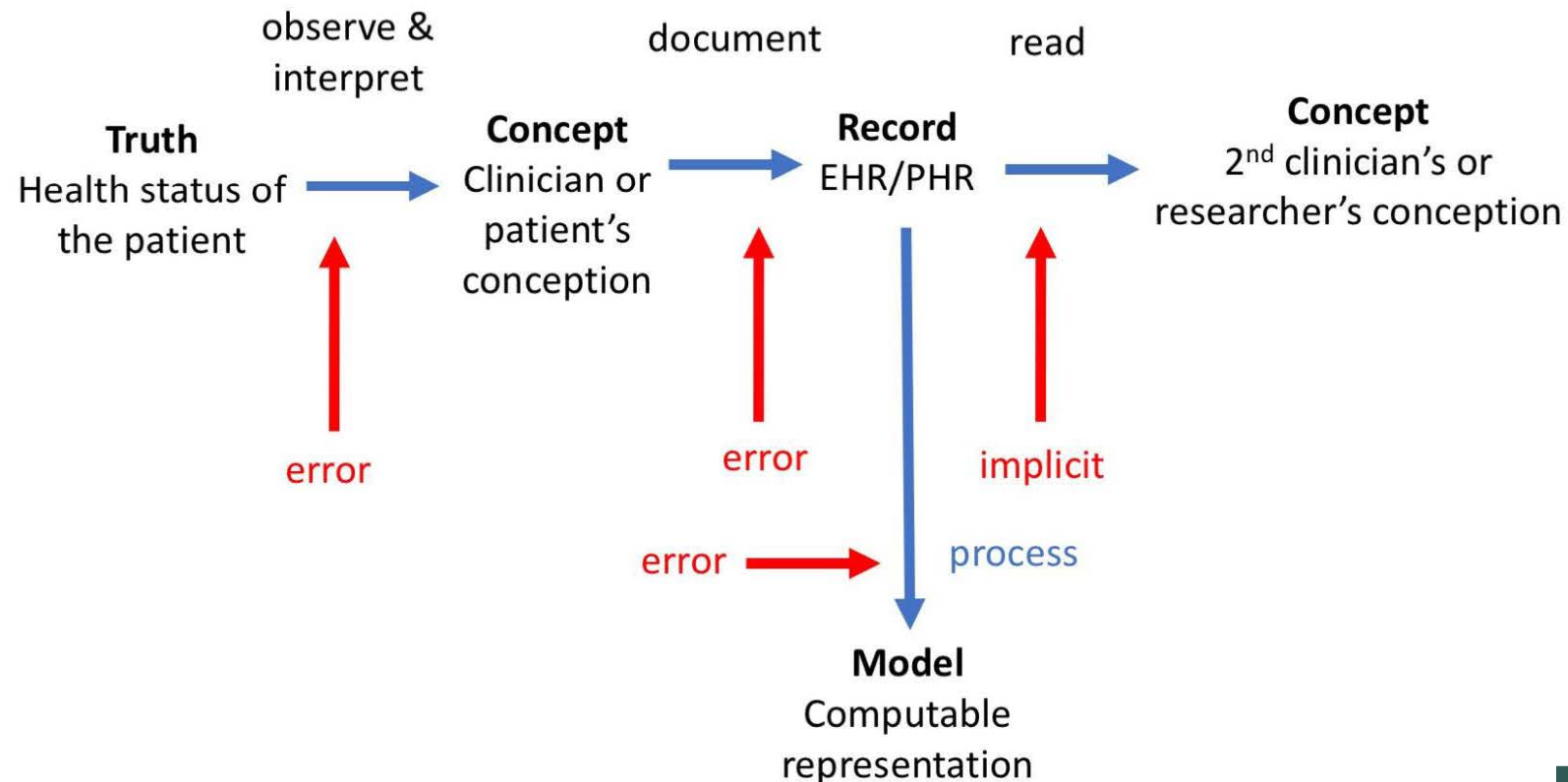
Source: Greg Simon, MD, Group Health Research Institute

# Longitudinal data linkage

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



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

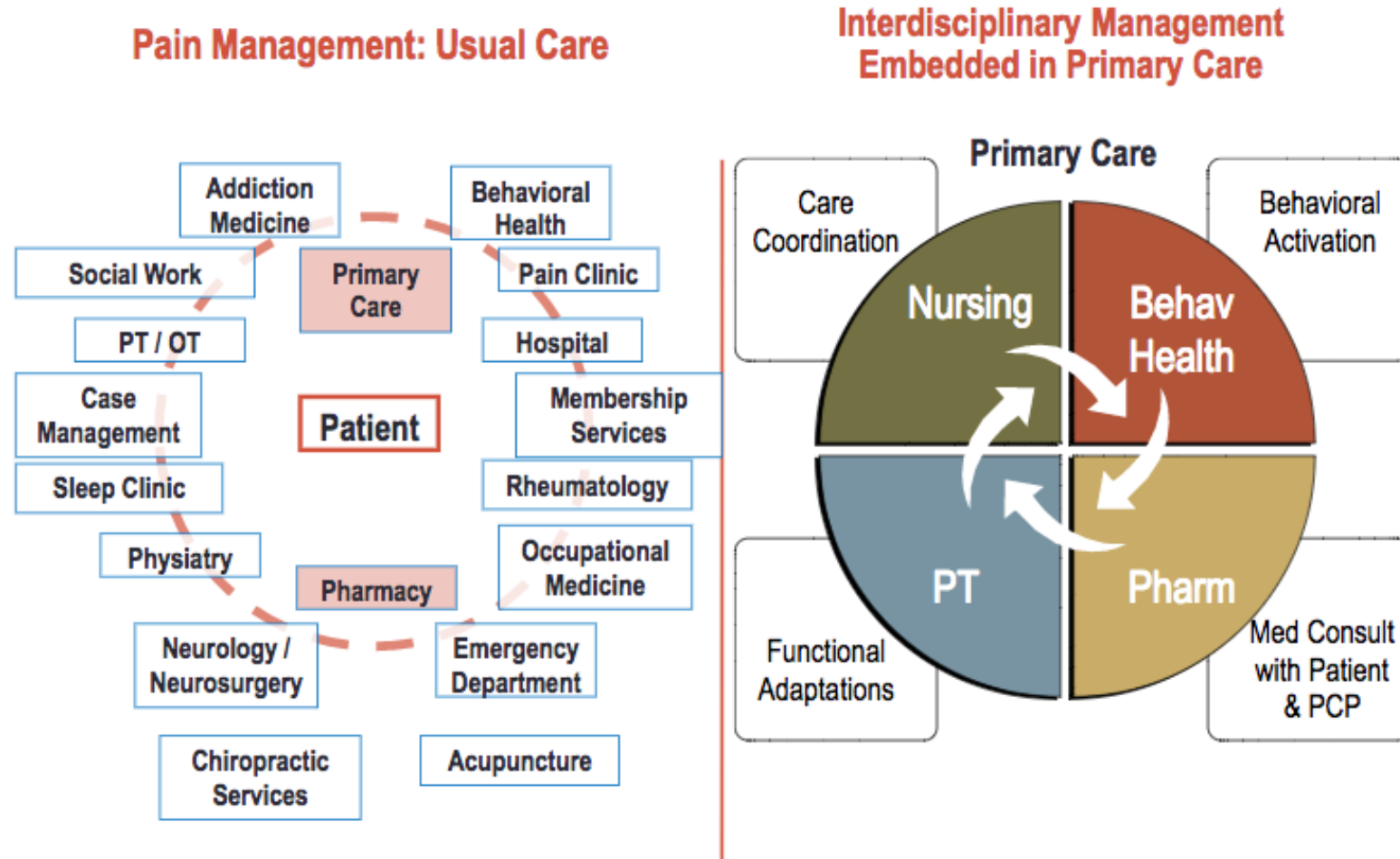


*For example, a trial measuring outcomes that matter most to patients and health systems may not be able to rely exclusively on information from the EHR, and instead need to assess patient-reported outcomes, which is more expensive and less efficient*

# 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)

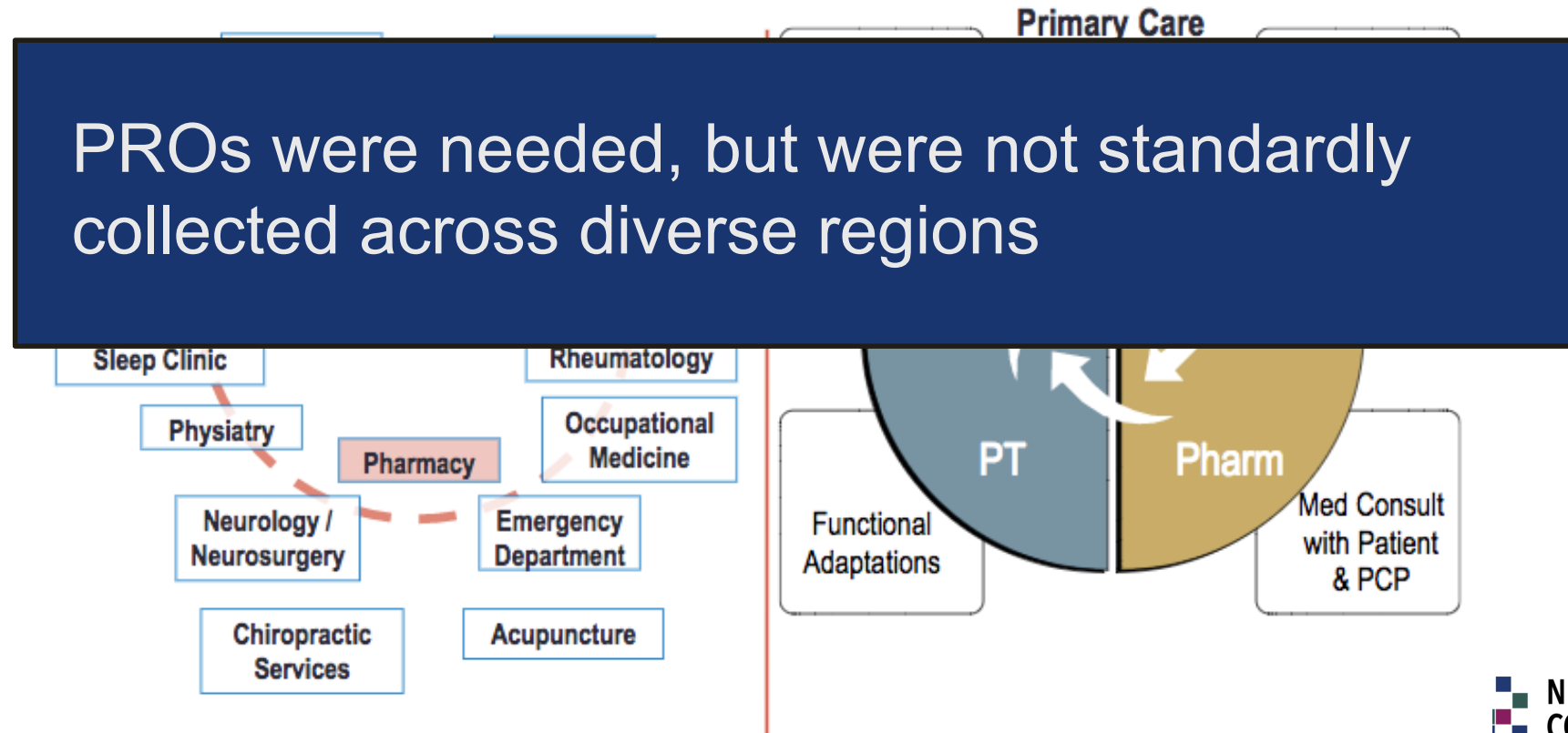


Source: Lynn DeBar, PhD, MPH, Kaiser Permanente Washington Health Research Institute

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

Pain Management: Usual Care

Interdisciplinary Management  
Embedded in Primary Care

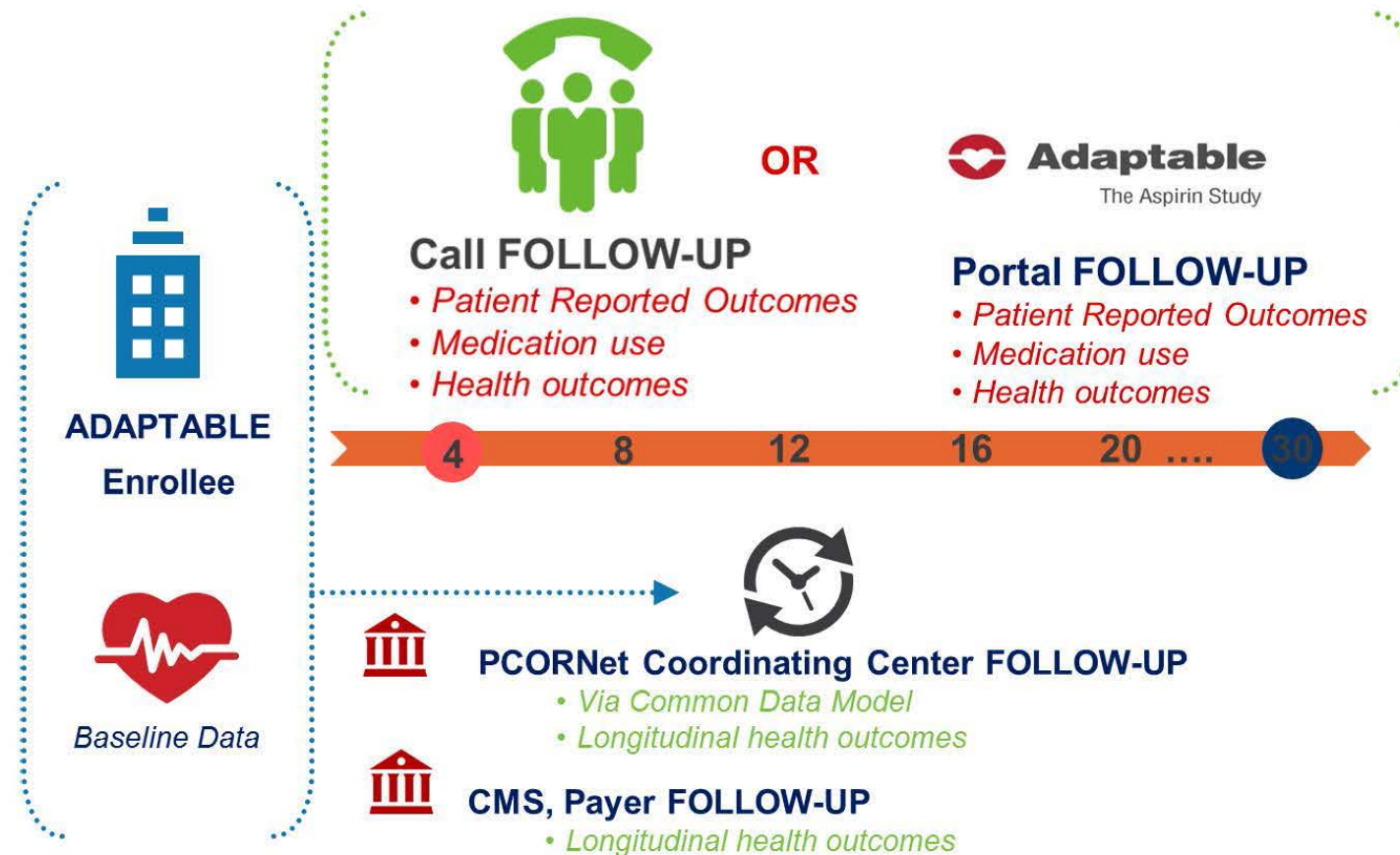


Source: Lynn DeBar, PhD, MPH, Kaiser Permanente Washington Health Research Institute

# 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

# 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



# A Health Equity Lens

- “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:**

Incomplete and variable capture of data on social determinants of health

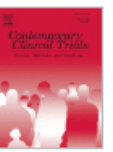
Lack of representation from vulnerable populations that do not access or receive treatment

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](#),<sup>✉</sup> [Rosa Gonzalez-Guarda](#), [Katharine Lawrence](#), [Crystal L Patil](#),

Data loss due to variable use of technology

# A Health Equity Lens



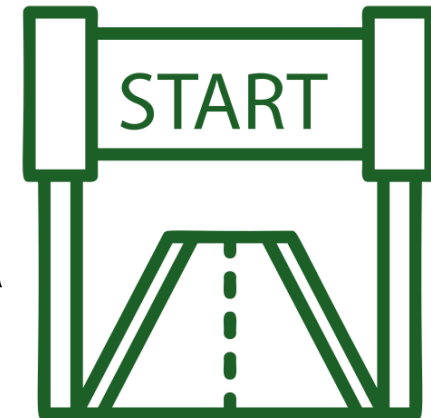
Short Communication

## Equity and bias in electronic health records data

Andrew D. Boyd <sup>a</sup>, Rosa Gonzalez-Guarda <sup>b</sup>, Katharine Lawrence <sup>c</sup>,

### 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



# Integrating health equity in outcomes

- Select outcomes relevant to health disparity populations
- Assess the validity of outcomes for health-equity-relevant groups to ensure applicability in specific populations
- Explore how outcomes may be interpreted with respect to norms and expectations of health-equity-relevant groups
- Consider pilot work to evaluate acceptability and psychometrics of outcomes for health disparity populations
- Require linguistic and health literacy accessibility in outcome ascertainment
- Ensure health equity of outcome data capture

## Best Practices for Integrating Health Equity into Embedded Pragmatic Clinical Trials for Dementia Care



### 6 Best Practices for **Selecting Outcomes**

*Integrating Health Equity into ePCTs for Dementia Care*



- 1** Select outcomes relevant to health disparity populations  
Select outcomes with evidence of broad value in health disparity populations.
- 2** Assess the validity of outcomes for health-equity-relevant groups to ensure applicability in specific populations  
Assess existing evidence of outcome validation regarding race, ethnicity, educational attainment and other health-equity-relevant characteristics.
- 3** Explore how outcomes may be interpreted with respect to norms and expectations of health-equity-relevant groups  
Review qualitative and comparative research on cultural differences in the lived experience of people living with dementia (PLWD), and the meaning of potential outcome domains.
- 4** Consider pilot work to evaluate acceptability and psychometrics of outcomes for health disparity populations  
In the absence of evidence of psychometric properties in specific populations, pilot work may be needed to assess an outcome measure's validity, reliability, and cross-cultural differences in these groups.

### How to Use this Packet

Health-equity-relevant considerations are necessary in all aspects of ePCTs. The key is to consider these issues early in the planning process, as well as systematically and throughout the conduct of the trial. Health-equity-relevant concepts can be nuanced and complex, and the degree to which researchers can incorporate health equity into each ePCT design component depends on the scope and objectives of the trial. These best practices are meant as a starting place for investigators to systematically explore how to integrate health equity into their ePCT design and identify potential pitfalls in their current research processes.

# 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
- 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

# 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](http://www.rethinkingclinicaltrials.org)

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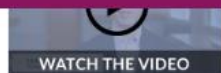
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What is a Pragmatic Clinical Trial?  
Endpoints and Outcomes  
Building Partnerships and Teams to Ensure a Successful Trial

Developing a Compelling Grant Application  
Analysis Plan  
Intervention Delivery and Complexity

Experimental Designs and Randomization Schemes  
Using Electronic Health Record Data



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.

What is a  
**PRAGMATIC CLINICAL TRIAL?** ▶

**TRAINING RESOURCES** ▶

# Question & Answer



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