NIH Pragmatic Trials Collaboratory 2026 Annual Workshop

Endpoint Strategy in Pragmatic Trials

From Big Questions to Meaningful Outcomes

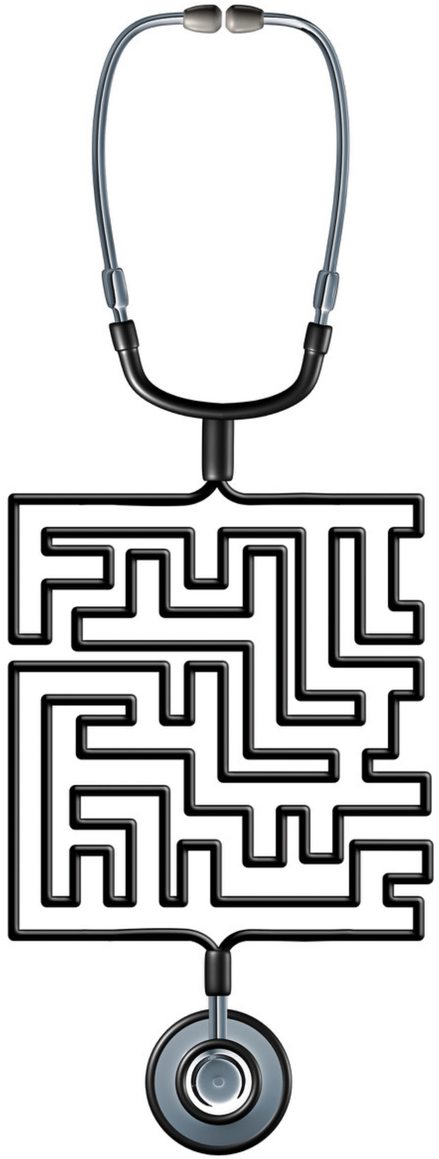
NIH PRAGMATIC TRIALS COLLABORATORY

Rethinking Clinical Trials®

Housekeeping

- Please pose your questions in the Q&A
- Slides and recordings will be posted on rethinkingclinicaltrials.org after the workshop

NIH PRAGMATIC TRIALS COLLABORATORY
Rethinking Clinical Trials®



Moving from Measurement to Meaning

Kevin Weinfurt, PhD
Duke University School of Medicine

2026 NIH Workshop

Day 1 Themes

“Sneaky topic” (Emily O’Brien)

- Looks like statistics, but really about values

Be intentional and explicit

- Proximate/distal
- Estimand framework
- Combining/weighting components in multicomponent endpoints

Successful endpoint strategizing requires a high-functioning, multidisciplinary team

- Estimand framework as scaffolding
- Statistical properties + relevance to decision-makers

Day 1 Themes

Trade-offs are required

Proximate vs distal, time to getting an answer

Richer understanding with multiple outcomes vs rigor/discipline of focus on few

Design endpoint to detect whether treatment does something vs show what it does and how well it does it

Opportunities for learning

Guidance for handling different intercurrent events

Guidance for navigating larger toolbox for handling multiple outcomes of interest

Ideas for Day 2

Move from choosing a meaningful outcome/estimand to judging the meaningfulness of an observed treatment effect

Meaningful to whom?

Meaningful how?

Noticeable/perceptible vs worthwhile

How do we deal with uncertainty?

Pragmatic clinical trials (PCTs) are *"designed for the primary purpose of informing decision-makers regarding the comparative balance of benefits, burdens, and risks of a biomedical or behavioral health intervention at the individual or population level."*

Califf RM, Sugarman J. 2015. Exploring the ethical and regulatory issues in pragmatic clinical trials. Clin Trials. 12:436–441. doi:10.1177/1740774515598334. PMID: 26374676.

Ideas for Day 2

Move from choosing a meaningful outcome/estimand to judging the meaningfulness of an observed treatment effect

Meaningful to whom?

Meaningful how?

Noticeable/perceptible vs worthwhile

How do we deal with uncertainty?

What's a meaningful effect at the population- versus individual-level?