Opportunities for Embedded Pragmatic Clinical Trials (ePCTs)

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Disclosures

- Dr. Wendy Weber has no financial disclosures to report. The views expressed in this presentation are those of the speaker and do not necessarily reflect the position or policy of the NIH or the US government.

Learning goals

- Identify key considerations in design and conduct of ePCTs and how they differ from explanatory trials
- Learn about the advantages and disadvantages of ePCTs and when they can be used to answer research questions
- Introduce the intersection between ePCTs and innovative information capabilities

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Important things to know

ePCTs are designed to answer important, real-world clinical questions

Broad engagement and support are essential from beginning to end

Trade-offs in flexibility, adherence, and generalizability are inevitable

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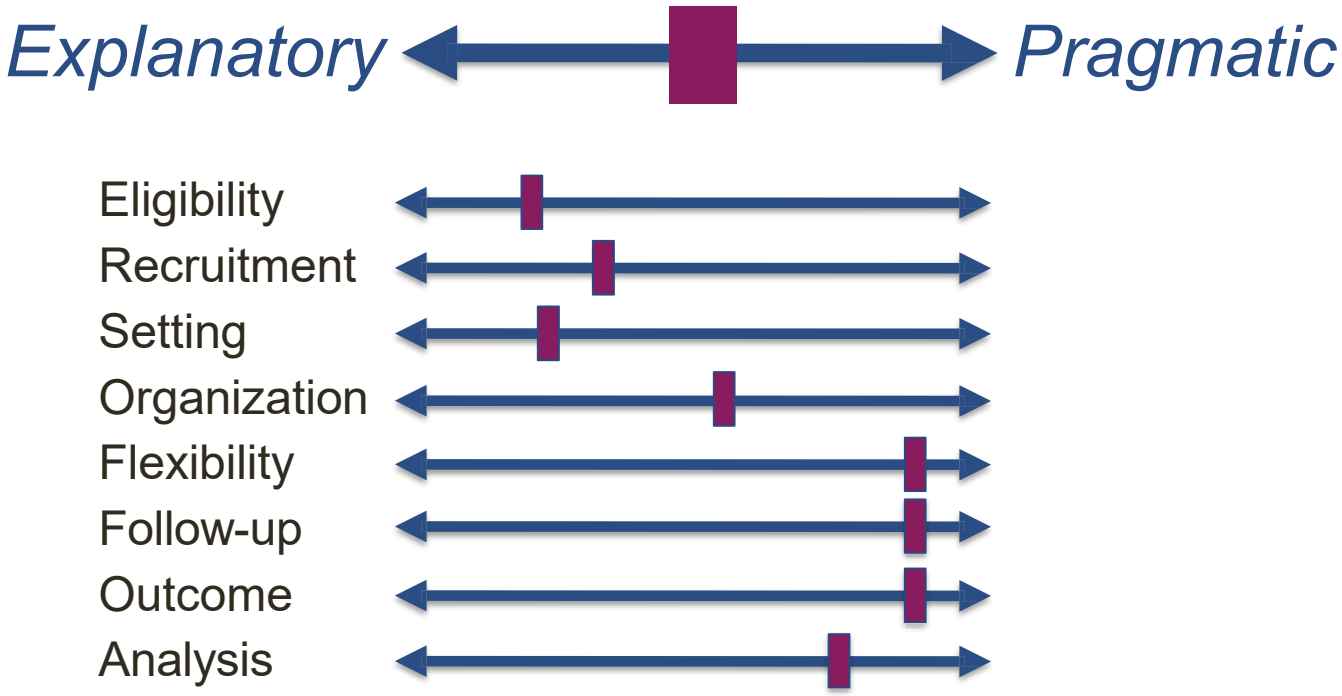
Trials elements vary across a spectrum



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Why conduct ePCTs?

- ☑ Potential to inform policy and practice with high-quality evidence

vs traditional trials

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ePCT characteristics

- Conducted within healthcare systems
- Use streamlined procedures and existing infrastructure
- Answer important medical questions

Why Do an ePCT? The 5 Rs

Relevant Question

The question is pressing, and healthcare system leaders, patients, and front-line clinicians care about the answer.

Real-World Setting

Desire to test in diverse healthcare delivery settings with the hope of implementing findings widely.

Representative Population

Ability to recruit a population reflective of patients with the condition, including those from minoritized communities.

Routinely Collected Data

Can use data collected as part of healthcare delivery to answer the question, supplemented by data from other sources.

Rigorous Methods

Randomized research is needed to answer the question and inform changes in care, policy, or reimbursement.

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Interventions tested in ePCTs are intentionally designed to align with health system priorities and leverage existing healthcare system infrastructure and resources, with the goals of easing intervention implementation during the trial and increasing the likelihood that effective interventions will be translated into routine practice posttrial.

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Problems

While many effective therapies exist, implementation is slow, ineffective, and unequal

- The delay comes at enormous cost to patients, payers, and manufacturers
- If Implementation is considered, it is late in the development process
- If implementation is not done well, it can make healthcare inequities worse

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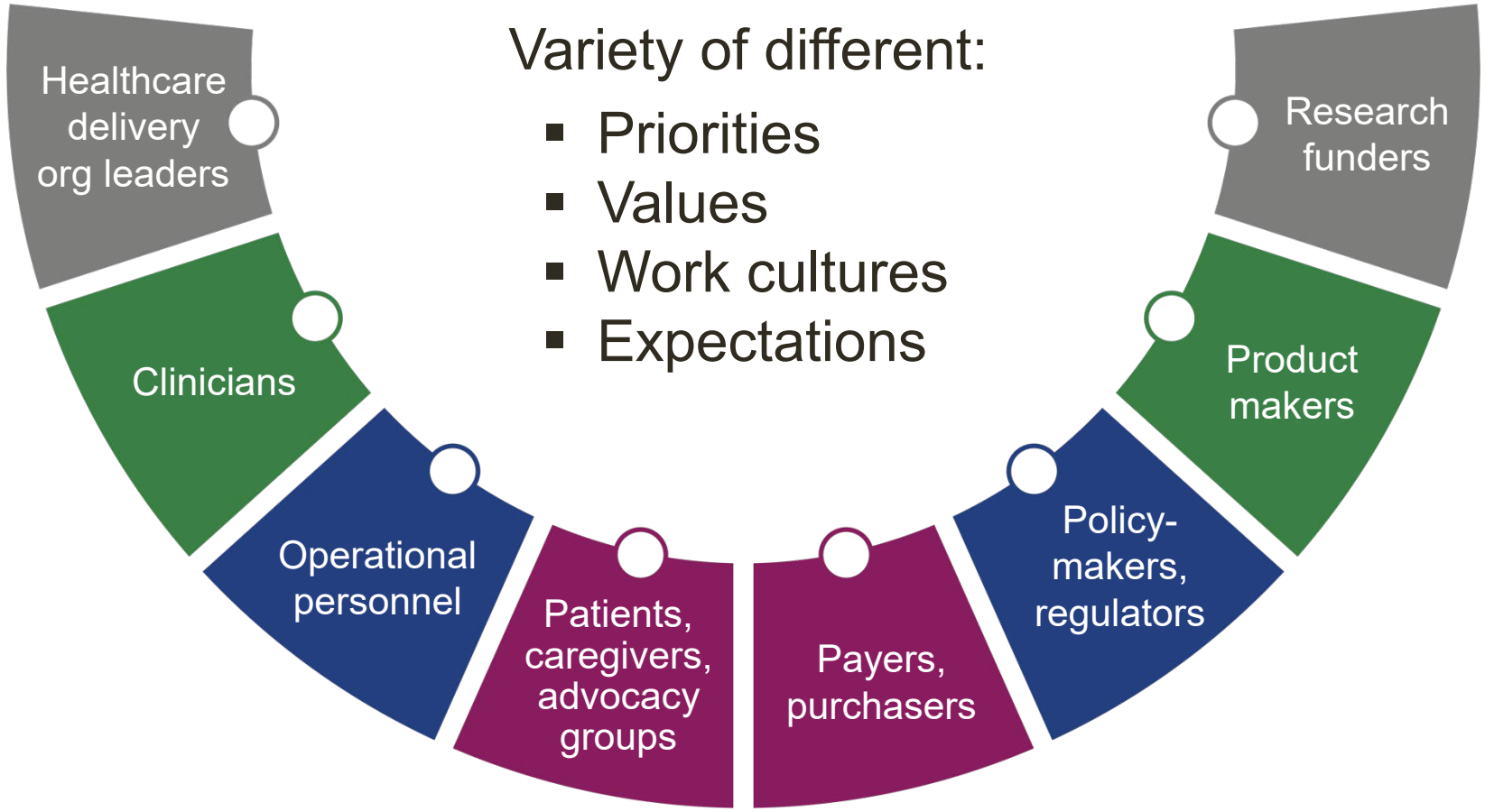
Who is interested and involved?



Variety of different:

- Priorities
- Values
- Work cultures
- Expectations

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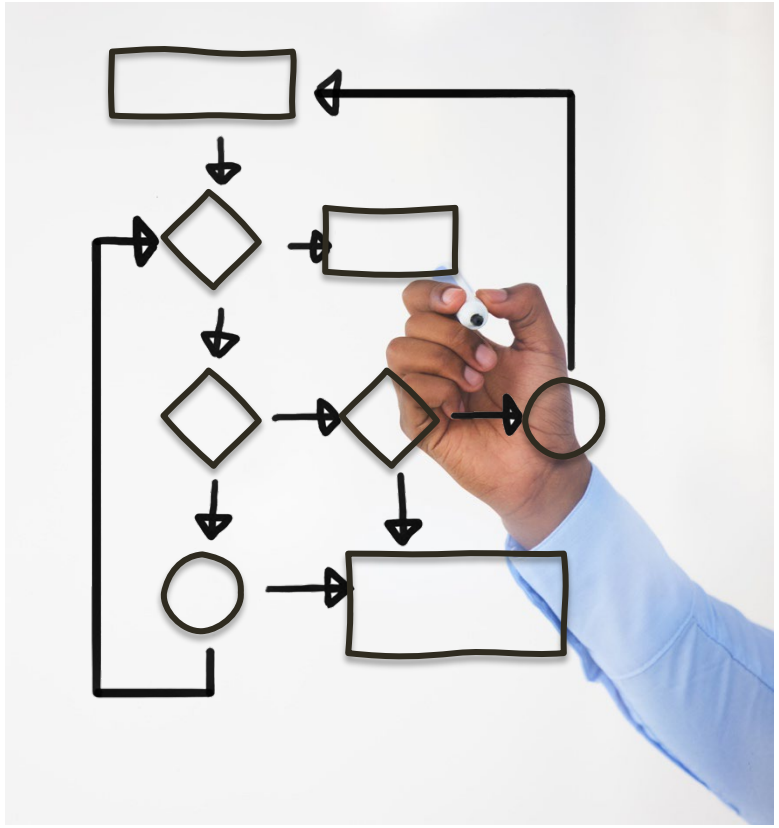
Use existing workflows



"The more complicated the intervention is to the existing workflow, the more difficult it is to get compliance—you can't just add on a new thing, you have to change what happens on the floor.

—Vincent Mor, PhD (PROVEN)

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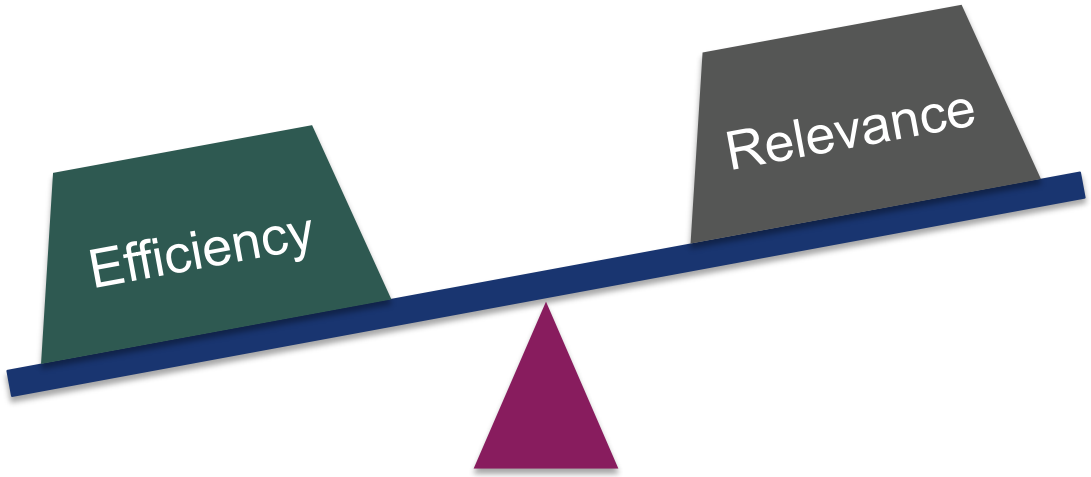
It’s a balancing act



ePCTs want to achieve both

But…high relevance to real-world decision-making may come at the expense of trial efficiency

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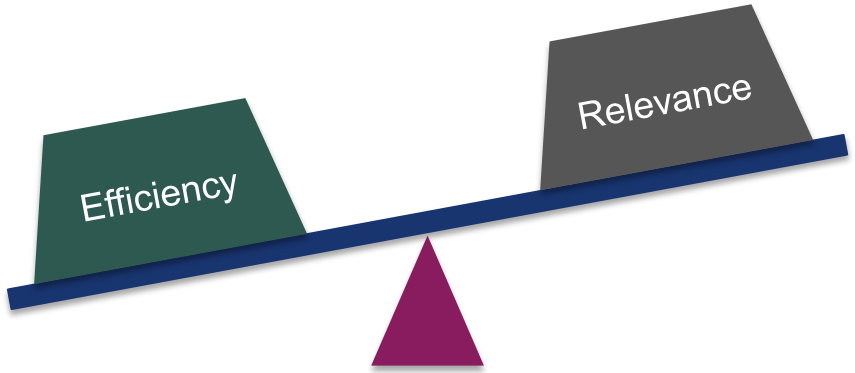
Example



Trial seeks to measure outcomes that matter most to patients and health systems

+

Information needed not available from the EHR

=

Must assess patient-reported outcomes, which are more expensive and less efficient

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ePCTs and innovative information capabilities

- Plan for infrastructure and expertise needs when incorporating innovative technologies and information capabilities into a trial
- Understand the ways ePCTs are well-suited for evaluating implications of innovative technologies for clinical care delivery
- Consider how the latest information capabilities, such as AI, can enhance ePCTs, and think through the ethical considerations

Important things to do

- Set expectations to work collaboratively and build trust from the beginning
- Get to know your partners' values, priorities, and expectations
- Assess your partners' capacity and capabilities
- Track goals reached, challenges, and adaptations throughout the life cycle of your ePCT
- Show appreciation and celebrate accomplishments early and often to have sustained partnerships

Question & Answer

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Knowledge checkpoint

- Which of the following are common design elements of embedded pragmatic clinical trials?
  1. Interventions delivered by clinicians or other providers already in the health care setting
  2. Enrollment criteria for participants are broad to increase generalizability
  3. Data from electronic health records are leveraged for some of the study outcomes
  4. All of the above

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Knowledge checkpoint

- True or False: Researchers know the most important questions to ask in clinical trials and it doesn't matter if the health care system partner thinks the research is unimportant.

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Knowledge checkpoint

- True or False: Implementation science methods and strategies can improve the conduct of embedded pragmatic clinical trials.