What Are Embedded PCTs?

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Learning goals

- Identify key considerations in the design and conduct of ePCTs and how they differ from explanatory trials
- Learn about the advantages and disadvantages of ePCTs, when a pragmatic approach can be used to answer the research questions

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Important things to know

- ePCTs are designed to answer important, real-world clinical questions
- Broad stakeholder engagement and support are essential from beginning to end
- Trade-offs in flexibility, adherence, and generalizability are inevitable

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90%

By 2020, 90% of clinical decisions should be supported by accurate, timely, and up-to-date information based on the best available evidence.

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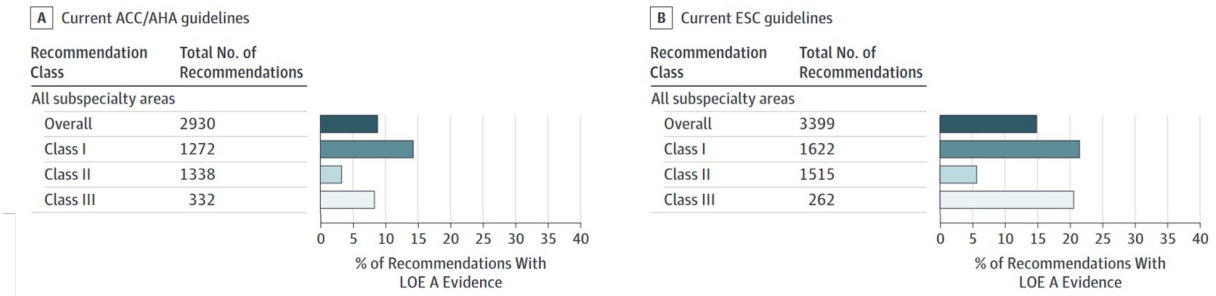
What % of recommendations in current ACC/AHA and ESC guidelines are Level A*



Fanaroff AC. JAMA. 2019;321(11):1069-1080

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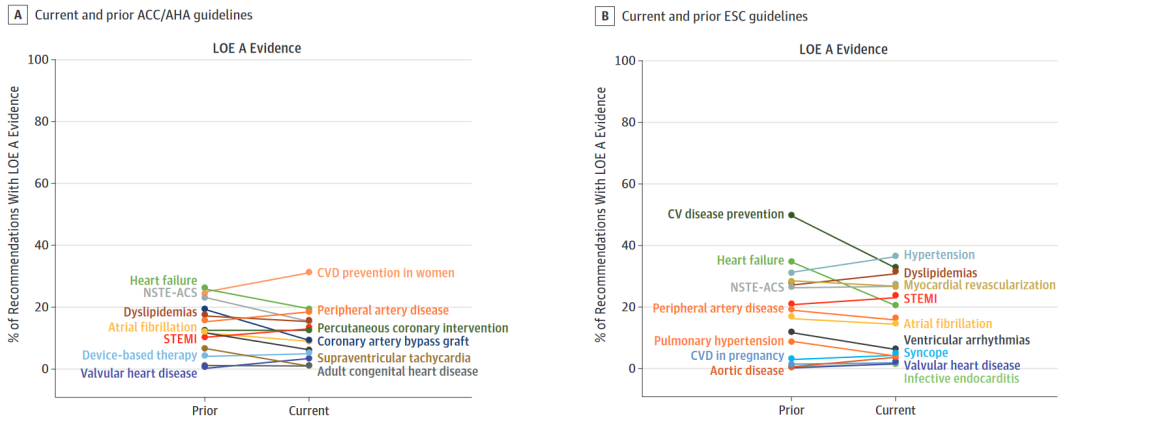
Are we getting better with time?



Prior 1999-2014
Current 2008-2018

Fanaroff AC. JAMA. 2019;321(11):1069-1080

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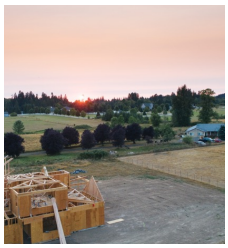
If healthcare were like…

Banking

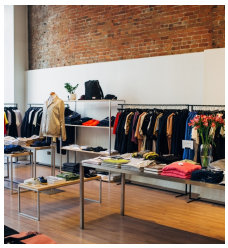
ATM transactions would take not seconds but perhaps days or longer as a result of unavailable or misplaced records.

Home building



Carpenters, electricians, and plumbers each would work with different blueprints, with very little coordination.

Shopping

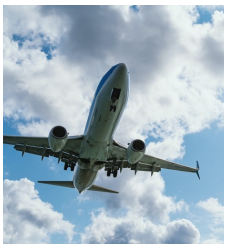


Product prices would not be posted, and the price charged would vary widely within the same store, depending on the source of payment

Car manufacturing

Warranties [covering] defects would not exist...so few factories would seek to monitor and improve production line performance and product quality

Airline travel



Each pilot would be free to design his or her own preflight safety check, or not to perform one at all

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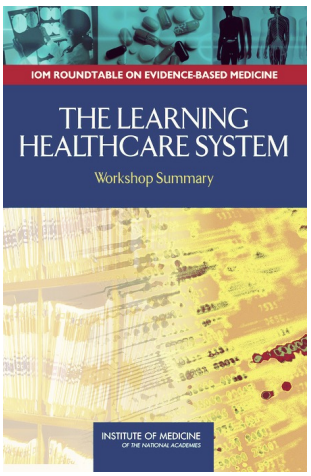
Change requires “leaders to consider rigorous evidence generation a core function of ordinary health care, research funders to prioritize practical questions relevant to population health and to support infrastructure for embedded research.”



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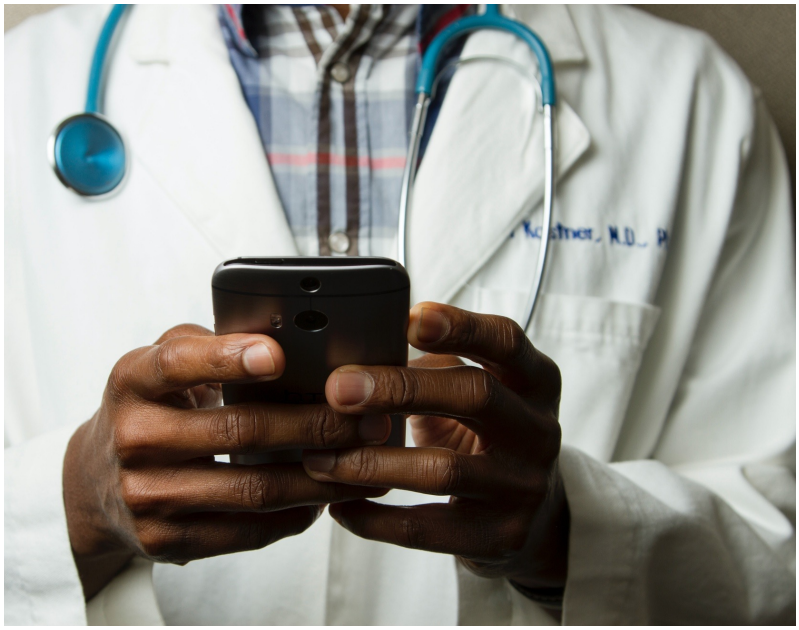

PCTs have the potential to inform policy and practice with high-quality evidence at reduced cost and increased efficiency compares with traditional clinicals.

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What makes a trial pragmatic?

Conducted within the healthcare system

Not disruptive to clinical workflow

Streamlined procedures and infrastructure

Makes use of existing data

Answer questions with major public health importance

Outcomes important to decision-makers

Diverse, representative study population

Highly generalizable results

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Internal vs. External Validity

Explanatory trials aim for high internal validity

Strict controls and procedures maximize validity of results within the study context

PCTs aim for high external validity

Real-world settings and data sources improve applicability of findings to broader context

Tradeoff between control and realism

More control improves internal validity but may reduce generalizability

Different trial designs have inherent tradeoffs between internal and external validity

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"Explanatory trials tell you what is true. Pragmatic trials tell you what to do."
Greg Simon, MD, MPH

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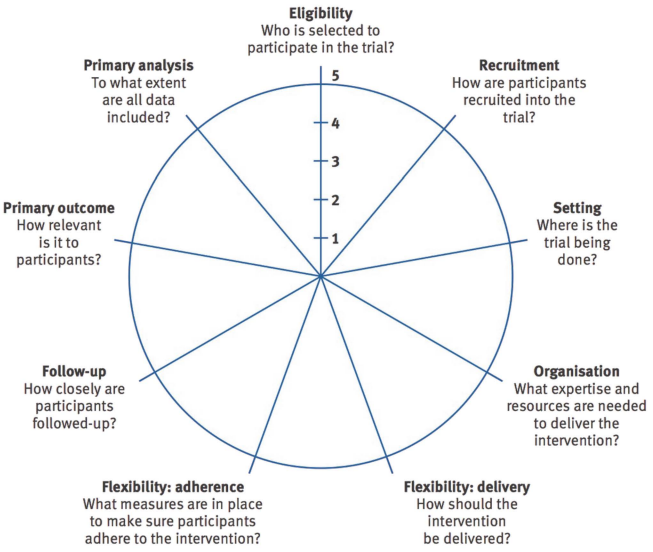

The **PRECIS-2 tool** helps trialists consider where they would like their trial to be on the pragmatic/explanatory continuum

PRECIS=Pragmatic Explanatory Continuum Indicator Summary

https://www.precis-2.org

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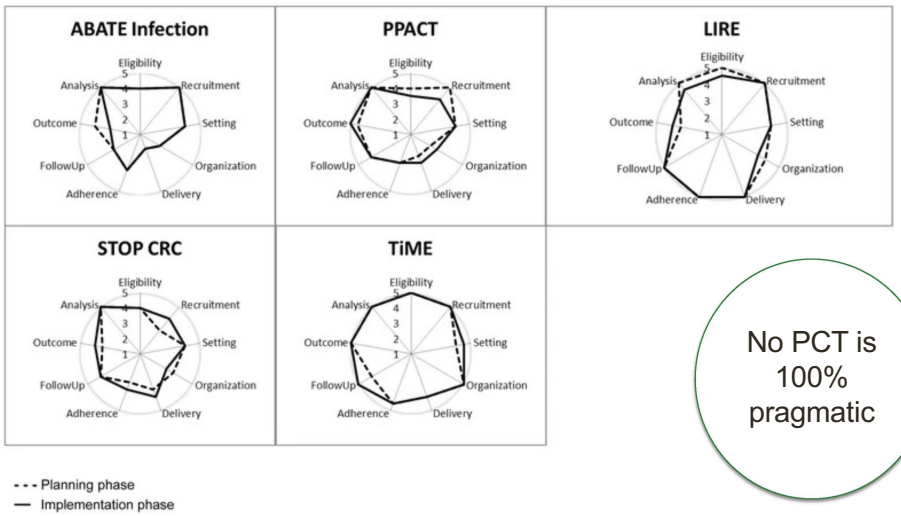

No PCT is 100% pragmatic

Johnson, KE, et al. Trials **17**, 32 (2016)

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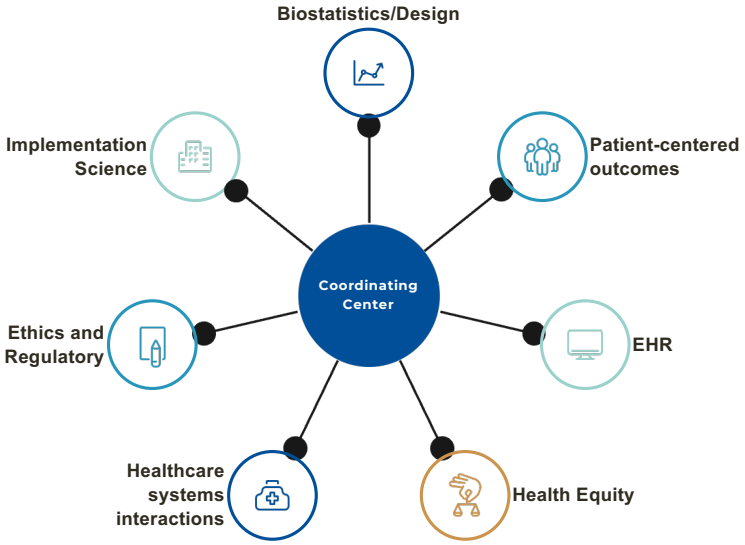

NIH Pragmatic Trials Collaboratory

A centralized Coordinating Center and the Core groups assist with the design, conduct, and logistical challenges of the projects and disseminate generalizable knowledge about PCT methods and enabling factors

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32 large-scale, high impact demonstration projects (PCTs)

Multi-phase
Planning→ implementation phase

NIH-supported
Span multiple institutes and offices

Diverse topics
Ranging from hospital-based infections, chronic pain, opioid use, medication adherence, and suicide

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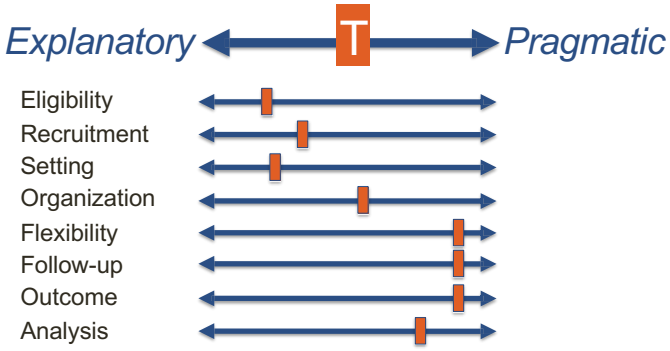
Trials vary across a spectrum of explanatory and pragmatic elements

Different trial elements are, by design, more or less explanatory/pragmatic



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Why conduct ePCTs?

ePCTs have the potential to inform policy and practice with high-quality evidence at reduced cost and increased efficiency compared with traditional clinical trials

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ePCT characteristics

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

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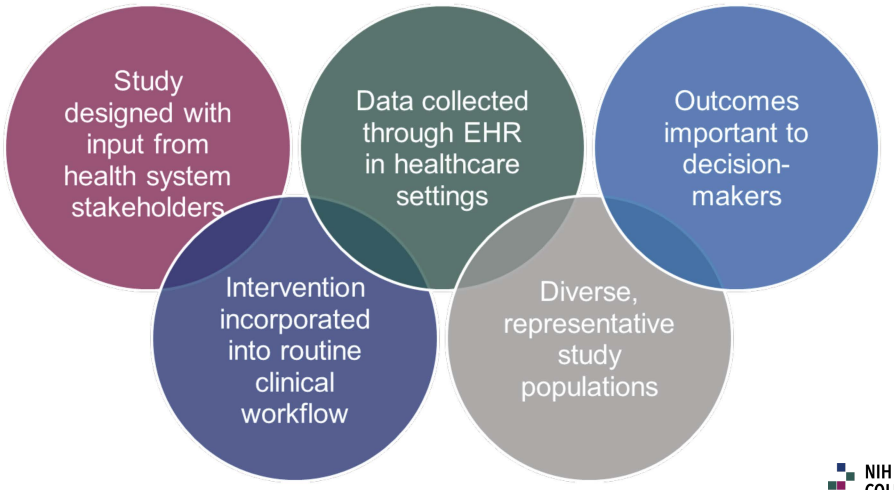
ePCTs bridge clinical care into research



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Who are your stakeholders?

Potential stakeholders have a variety of priorities, values, work cultures, and expectations:

- Healthcare delivery organization leaders
- Clinicians
- Operational personnel
- Patients, caregivers, patient advocacy groups
- Payers, purchasers
- Policy makers, regulators
- Research funders
- Researchers
- Product manufacturers

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Listen to the frontline

The purpose of the healthcare system is not to do research, but to provide good healthcare. Researchers often have a tail-wagging-the-dog problem. We assume if we think something is a good idea, the healthcare system will too… We need to remember that we're the tail and the healthcare system is the dog.

– Greg Simon, MD, MPH (SPOT)

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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

Achieving both relevance and efficiency is a goal of pragmatic trials, yet high relevance to real-world decision-making may come at the expense of trial 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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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 lifecycle of your ePCT
- Show appreciation and celebrate accomplishments early and often to have sustained partnerships

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Resource: The Living Textbook

Visit the Living Textbook of Pragmatic Clinical Trials at

www.rethinkingclinicaltrials.org

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Question & Answer

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