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Pragmatic Trials

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Pneumology

Pragmatic Trials

- A pragmatic clinical trial (PCT) is a clinical trial that focuses on correlation between treatments and outcomes in real-world health system practice rather than focusing on proving causative explanations for outcomes.
- The ideal time to perform a pragmatic trial would not be at the beginning, and more to help find the best setting in the treatment of a disease and to understand what the effect might be on overall public health.
- Therapy/treatment/management optimisation trials

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Table 1. Nine Dimensions for Assessing the Level of Pragmatism in a Trial, as Proposed in the Pragmatic–Explanatory Continuum Indicator Summary 2 (PRECIS-2) Tool.*

Dimension	Assessment of Pragmatism
Recruitment of investigators and participants	
Eligibility	To what extent are the participants in the trial similar to patients who would receive this intervention if it was part of usual care?
Recruitment	How much extra effort is made to recruit participants over and above what would be used in the usual care setting to engage with patients?
Setting	How different are the settings of the trial from the usual care setting?
The intervention and its delivery within the trial	
Organization	How different are the resources, provider expertise, and organization of care delivery in the intervention group of the trial from those available in usual care?
Flexibility in delivery	How different is the flexibility in how the intervention is delivered from the flexibility anticipated in usual care?
Flexibility in adherence	How different is the flexibility in how participants are monitored and encouraged to adhere to the intervention from the flexibility anticipated in usual care?
The nature of follow-up	
Follow-up	How different is the intensity of measurement and the follow-up of participants in the trial from the typical follow-up in usual care?
The nature, determination, and analysis of outcomes	
Primary outcome	To what extent is the primary outcome of the trial directly relevant to participants?
Primary analysis	To what extent are all data included in the analysis of the primary outcome?

* Information in the table is adapted from Loudon et al.[22]

Ford I, Norrie J. N Engl J Med 2016; 375:454-463

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- “The sponsor should ensure that all aspects of the trial are operationally feasible and should avoid unnecessary complexity, procedures and data collection.”

  ICH Harmonised guideline good clinical practice (GCP) E6(R3). Draft version, endorsed on 19 May 2023

- Selective safety data collection

  ICH guideline E19, 16 March 2023

- FDA 2022 Project Pragmatica

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- Good approach for getting more relevant data with less bureaucratic efforts, increased speed and less costs

  if

  – the rights, dignity, wellbeing and safety of patients

  – integrity of trial data and adequate evidence-generation

  are preserved

- → careful selection of the research topic, scientific and ethical advice

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Thank you!

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