YOUR STUDY DESIGN FEATURE(S)

ACCORDS
UNIVERSITY OF COLORADO
CHILDREN'S HOSPITAL COLORADO

Worksheet #2

Selecting Pragmatic Research Study Design Features

Use this worksheet with the following sessions:

⇒ Day 1 Keynote on Pragmatic Research

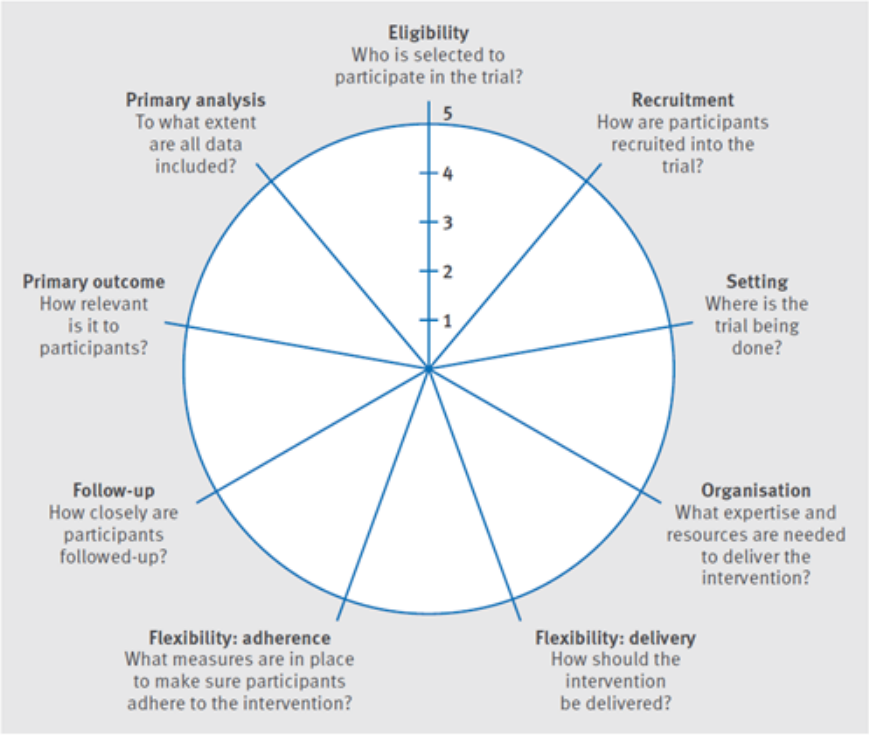
⇒ Day 1 Plenary on Planning Pragmatic Research and the PRECIS-2 framework

⇒ Day 1 Plenary on the Multiphase Optimization Strategy (MOST) Approach

⇒ Day 1 Tour of Pragmatic Study Design and Panel Discussion

The Pragmatic-Explanatory Continuum Indicator Summary (PRECIS-2)

The PRECIS-2 framework can be used a) as a study planning tool, b) to report on studies, and c) to rate the pragmatism of published studies as part of a systematic review. This latter approach may assist the selection of potential pragmatic and effective interventions. The PRECIS-2 has nine domains reflecting key design features of clinical trials. Each element of a study design is given a rating between 1 and 5 on each domain relative to usual care, with 1 representing a very explanatory (or efficacy-focused) trial and 5 representing a very pragmatic trial. For interactive rating tools, see the PRECIS-2.org website.



The PRagmatic-Explanatory Continuum Indicator Summary 2 (PRECIS-2) wheel.

Continued next page

YOUR STUDY DESIGN FEATURE(S)

ACCORDS
UNIVERSITY OF COLORADO
CHILDREN'S HOSPITAL COLORADO

Worksheet #2 continued

PRECIS-2 domains and ratings for your study		
PRECIS-2 domain	My Study Design	Pragmatic-Explanatory Rating
Eligibility criteria		
Recruitment path		
Setting		
Organization		
Flexibility: delivery		
Flexibility: adherence		
Follow-up		
Primary outcome – relevance to participants		
Primary analysis		

Continued next page

YOUR STUDY DESIGN FEATURE(S)

ACCORDS
UNIVERSITY OF COLORADO
CHILDREN'S HOSPITAL COLORADO

Worksheet #2 continued

Selecting Pragmatic Study Design Types

Use the following questions to help determine appropriate study design types for your pragmatic research. When we refer to an intervention, we mean any program, treatment, service or policy that will be tested in the setting in which it is intended to be used or delivered. *Contact a biostatistician (and possibly other experts such as a health economist; qualitative analysis expert; social network or systems analyst) early to discuss appropriate study designs and analytic techniques.*

- Will your design type be:

☐ Participant-level randomized trial

☐ Cluster randomized trial (level of randomization: _____; level(s) of outcome data: _____)

☐ Stepped wedge design (level of randomization to rollout: _____)

☐ Quasi-experimental design (Type: _____)

☐ Observational design (Type: _____)

☐ Factorial (full or partial) design

☐ SMART design

☐ Adaptive design

Comments:

- Will your study be focused on effectiveness only, implementation only, or both effectiveness and implementation outcomes (suggesting a hybrid trial may be appropriate)?

☐ Clinical effectiveness trial only (no implementation outcomes)

☐ Implementation trial only (no health outcomes)

☐ Hybrid Type I: Primary aim: clinical effectiveness (secondary aim: context for implementation, acceptability and feasibility)

☐ Hybrid Type II: Coprimary aims: clinical effectiveness and implementation (adoption, fidelity)

☐ Hybrid Type III: Primary aim: utility of an implementation strategy (secondary aim: clinical outcomes)

Comments:

Continued next page

YOUR STUDY DESIGN FEATURE(S)

ACCORDS
UNIVERSITY OF COLORADO
CHILDREN'S HOSPITAL COLORADO

Worksheet #2 continued

Selecting Pragmatic Study Design Types

To decide, consider the following:

- Is randomization to condition possible, ethical, and feasible? Why or why not?

- For non-randomized designs
  - Consider an observational, quasi-experimental design, or natural experiment.

- For randomized designs
  - Will randomization be at the participant level or provider/site/cluster level? Why?

    Consider a cluster randomized trial or stepped wedge design if there is the possibility of contamination or pragmatic challenges with participant level randomization (e.g., an organization or provider would be unable to deliver an intervention more than one way at a time due to resources)

  - Is the recruitment rate likely to be constant across time?

    If no, consider cluster randomized rather than stepped wedge to mitigate study delays when recruitment is low.

  - How feasible is it to implement the intervention for all randomization units at the same time?

    If not feasible, consider a stepped wedge to distribute the implementation at clusters at different time points.

  - Are more than two interventions being compared?

    If yes, consider a cluster randomized trial or a participant-level randomized trial instead of a stepped wedge design.

Continued next page

YOUR STUDY DESIGN FEATURE(S)

ACCORDS
UNIVERSITY OF COLORADO
CHILDREN'S HOSPITAL COLORADO

Worksheet #2 continued

Selecting Pragmatic Study Design Types

◊ Do the intervention(s) to be tested have multiple components that need to be optimized in terms of combination, sequence, dose, or tailoring?

If yes, an adaptive trial design (e.g., SMART) or factorial design may be appropriate. Also considered a MOST approach for iterative design and testing of an optimized intervention strategy.

♦ Other considerations

◊ Power and Sample Size Estimation

Pragmatic trials with an active comparator may anticipate a small effect size difference, which requires more participants to achieve adequate statistical power. What is your anticipated effect size difference for your study? Do you have access to the required sample size in your partnering sites?

◊ Analysis

Standard methods for analysis of individually randomized trials may not be appropriate. Statistical analysis must incorporate the study design features, such as hierarchical dependency of data and temporal trends. What analytic approach(es) might be appropriate?

Continued next page

YOUR STUDY DESIGN FEATURE(S)

ACCORDS
UNIVERSITY OF COLORADO
CHILDREN'S HOSPITAL COLORADO

Worksheet #2 continued

Key resources and references:

1. PRECIS-2 website: https://www.precis-2.org/
2. Loudon et al., The PRECIS-2 tool: designing trials that are fit for purpose: *BMJ* 2015;350:h2147.
3. Luoma K., Leavitt I. et al., How Can Clinical Practices Pragmatically Increase Physical Activity for Patients With Type 2 Diabetes? A Systematic Review. TBM, 2017.
4. Ali SA, Kloseck M, et al. Evaluating the design and reporting of pragmatic trials in osteoarthritis research. *Rheumatology (Oxford)*. 2018;57(1):59-63.
5. Brown CH, Curran G, et al. Overview of Research and Evaluation Designs for Dissemination and Implementation. *ARPH*, 2017
6. Thorlund K, Haggstrom J, et al.. Key design considerations for adaptive clinical trials: a primer for clinicians. *BMJ*, 2018
7. Curran GM, Bauer M, et al.. 2012. Effectiveness-implementation hybrid designs: combining elements of clinical effectiveness and implementation research to enhance public health impact. *Med. Care* 50:217–26
8. Brown et al 2017. An Overview of Research and Evaluation Designs for Dissemination and Implementation. *Annual Review of Public Health*
9. Hussey MA, Hughes JP. Design and analysis of stepped wedge cluster randomized trials. *Contemp Clin Trials*. 2007;28(2):182-191.
10. Hemming K, Lilford R, Girling AJ. Stepped-wedge cluster randomised controlled trials: a generic framework including parallel and multiple-level designs. *Statist Med*. 2015;34(2):181-196.
11. Campbell, M.K., Mollison, J. and Grimshaw, J.M. (2001), Cluster trials in implementation research: estimation of intracluster correlation coefficients and sample size, Statistics in Medicine, 20, 391-399.
12. Campbell MJ, Donner A, Klar N. Developments in cluster randomized trials and *Statistics in Medicine*. Statistics in Medicine. 2007 Jan 15;26(1):2-19.
13. Eldridge, S.M, Ashby, D., Feder, G.S., Rudnicka, A.R. and Ukomunne, O.C. (2004) Lessons for cluster randomized trials in the twenty-first century: a systematic review of trials in primary care Clinical Trials 1:80-90