Clinical trial design

see https://www.ncbi.nlm.nih.gov/pmc/articles/PMC3083073/

Classification

Clinical trial designs can be classified into several categories based on their structure, purpose, and methodology. Here are some common classifications:

Based on Structure:

Parallel (Non-crossover) Design: Participants are randomly assigned to different treatment groups, and each group receives a different intervention throughout the study without switching. Crossover Design: Participants receive multiple interventions or treatments in a sequential order, with each participant serving as their own control. Based on Purpose:

Efficacy Trials: Designed to evaluate the effectiveness of an intervention under ideal conditions. Effectiveness (Pragmatic) Trials: Designed to assess how well an intervention works in real-world conditions. Exploratory Trials: Aimed at gaining insights into a new treatment or disease. Confirmatory Trials: Aimed at confirming the effects observed in earlier exploratory trials. Based on Phase:

Phase I: Primarily concerned with safety and dosage in a small group of participants.

Phase II: Investigates effectiveness and further assesses safety in a larger group.

Phase III: Compares the new treatment to the standard treatment in a large group to confirm effectiveness and monitor side effects.

Phase IV: Conducted after the treatment is on the market to monitor long-term effects and collect additional information.

Based on Randomization:

Randomized Controlled Trial (RCT): Participants are randomly assigned to different treatment groups.

Non-Randomized (Observational) Trial: Participants are not randomly assigned, and the assignment is based on other factors.

Based on Blinding:

Blind (Single-blind, Double-blind): Participants or researchers are unaware of the treatment assignments to minimize bias.

Open-label: Both researchers and participants know the assigned treatments.

Based on Endpoint:

Superiority Trial: Designed to show that the new treatment is better than the standard treatment.

Non-inferiority Trial: Designed to show that the new treatment is not worse than the standard

treatment by more than a predetermined amount.

Equivalence Trial: Designed to show that the new treatment is as effective as the standard treatment.

Based on Duration:

Cross-Sectional Studies: Data collected at a single point in time.

Longitudinal Studies: Data collected over an extended period.

Understanding these classifications helps researchers design studies that answer specific questions about the safety and efficacy of interventions. Each type of trial design has its strengths and limitations, and the choice depends on the research question and practical considerations.

Terms in clinical trial design

Alternative Hypothesis.

Intent-to-treat (ITT)

Null Hypothesis

Phase 1

Phase 2

Phase 3

Phase 4

Power

The probability of rejecting a null hypothesis when it should be rejected. In superiority trials (e.g., trials designed to show that a new treatment is superior to placebo) this means the probability of identifying a treatment effect when indeed a true treatment effect exists.

Type I Error The probability if rejecting the null hypothesis when it should not be rejected (i.e., a false positive). In superiority trials this means the probability of (incorrectly) identifying a treatment effect when indeed a true treatment effect does not exist.

Type II Error The probability of failing to reject the null hypothesis when it should be rejected (i.e., a false negative). Type II error is the compliment of “power”. In superiority trials this means the probability of failing to identifying a treatment effect when indeed a true treatment effect exists.