Clinical Trial Cheatsheet

Phases of Clinical Trials

- Phase 0: Exploratory study (Micro-dosing) to check drug distribution in body. (Small N: < 15).
- Phase I: Safety assessment. Determines dose range and identifies side effects. (Healthy volunteers, N: 20-100).
- Phase II: Efficacy assessment. Evaluates if the drug works for the target disease. (Patient N: 100-300).
- Phase III: Comparative assessment. Compares new drug to current standard treatment or placebo. (Patient N: 300-3,000+).
- Phase IV: Post-marketing surveillance. Monitors long-term efficacy and rare side effects after approval.

Core Concepts and Terminology

- `Protocol`: A detailed plan describing the objective, design, and methodology of the trial.
- `Placebo`: An inactive substance that looks like the experimental drug.
- `Randomization`: Assigning participants to groups by chance to reduce bias.
- `Blinding`:
  - `Single-blind`: Participant doesn't know the group.
  - `Double-blind`: Neither participant nor researcher knows the group.
- `Inclusion / Exclusion Criteria`: Rules for who can or cannot participate in the trial.
- `Informed Consent`: The process where participants are fully informed about trial risks/benefits before agreeing to join.

Data Standards and Regulatory

- `GCP (Good Clinical Practice)`: International ethical and scientific quality standard for clinical trials.
- `CDISC (Clinical Data Interchange Standards Consortium)`:
  - `SDTM`: Standard for raw data tabulation.
  - `ADaM`: Standard for analysis datasets.
- `FDA (Food and Drug Administration)`: US regulatory body.
- `EMA (European Medicines Agency)`: EU regulatory body.
- `IND (Investigational New Drug)`: Application to start human trials.
- `NDA (New Drug Application)`: Application for marketing approval.

Statistical Analysis in Trials

- `Intention-to-Treat (ITT)`: Analyzing participants based on their initial group assignment, regardless of if they completed the trial.
- `Per-Protocol (PP)`: Analyzing only those who strictly followed the protocol.
- `Primary Endpoint`: The main outcome measured to determine trial success (e.g., survival rate).
- `Secondary Endpoints`: Additional outcomes measured for further insights.
- `Power`: The probability of detecting an effect if one truly exists.

Pro Tips

Adverse Events (AE)

An Adverse Event is any untoward medical occurrence in a patient, whether or not it's related to the treatment. If it's serious (death, life-threatening), it's labeled an SAE (Serious Adverse Event).

CRO (Contract Research Organization)

A company that provides support to the pharmaceutical, biotechnology, and medical device industries in the form of outsourced research services.

ClinicalTrials.gov

The major registry for clinical trials. Essential for tracking ongoing research and results.