Randomized Clinical Trials - Study Design II

MACE CODAY, PHD

PROFESSOR, PREVENTIVE MEDICINE, UTHSC

Outline

- The Question
- Evidence for Causality
- Specific Aims
- Name of Design – Randomized Clinical Trial
- Response Variables and Loss to Follow Up
- Eligibility Criteria
- Baseline Assessment
- Randomization/Blinding Procedures
- Alternative Designs

The Research Question

- The Scientific Question
- Feasibility/Timeliness
- Who, What, Where, Why

Why the Randomized Clinical Trial?

- RCT is Gold Standard Trial Design for Causality
  - Adding to evidence of causality
  - Applying study design procedures to reduce inherent bias
  - Random assignment and blinding procedures
  - The research question needs to be more than exploratory – where is the science?

Definitions

- **Specific Aims:**
  - Intervention Groups
  - Control Groups
  - Hypothesis Driven Group Selection

Definitions

- Name Your RCT Design
  - Efficacy
  - Effectiveness
  - Hybrid

Your Outcomes – The Data Collected

- Response Variables
  - Demographics
  - Objective Measures
  - Subjective Measures
  - Adverse Events
  - Serious Adverse Events

Eligibility Criteria

- Who Are You Recruiting in Versus Screening Out?
  - Who and Where?
  - Flow of Screening to Randomization
  - Figure 1 in Your Outcome Paper

Data Collection Tools

- Forms versus Electronic
  - Standardize Tools
  - Test the Measures
  - Simple versus Complex

Randomizing Procedures

Where to Start?

- Randomization Removes Biased Allocation to the Groups
- Randomization Usually Ensures Comparable Groups
- Randomization Guarantees the Validity of Statistical Tests of Significance

Types of Randomization

- Simple Randomization
- Block Randomization
- Stratified Randomization
- Alternative Randomizations

Blinding Procedures

- Types of Blinding to Remove Bias
  - Single
  - Double
  - Triple
- Blinding Reduces Biases
  - Known or Unknown
  - Measurement Error
  - Group Allocation Bias, Selection Bias

Alternative Trial Designs for the RCT

- Concurrent Non-Randomized
- Retrospective Historical Control
- Observational Cohort
- Within Group Cross-Over
- Dosing and Safety
- Pilot or Feasibility

Following Up and Tracking Adherence

- Recruitment Tracking/Adherence to Screening Procedures
- Visit Documentation/Adherence within Study Visits
- Retention Tracking/Adherence to Study Visits
- Treatment Adherence Documentation/Dose Adherence

Questions in General

Treatment Specific Questions

Questions Applicable to Your Research