

Randomized Clinical Trials - Study Design II

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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

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The Research Question

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

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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?

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Definitions

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

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Definitions

- ▶ Name Your RCT Design
 - ▶ Efficacy
 - ▶ Effectiveness
 - ▶ Hybrid

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Your Outcomes – The Data Collected

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

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Eligibility Criteria

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

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Data Collection Tools

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

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Randomizing Procedures

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Where to Start?

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

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Types of Randomization

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

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Blinding Procedures

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

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Alternative Trial Designs for the RCT

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

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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

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Questions in General

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Treatment Specific Questions

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Questions Applicable to Your Research

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