Randomized Clinical Trials
- Study Design II

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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