Randomized Controlled Trial

ADA Research Toolkit

ADA Research Committee
Learning Objectives

At the end of this presentation the participant will be able to:

- Define a randomized controlled trial and its components
- Describe the study design
- Describe the strengths and limitations of randomized controlled trials
- Identify the main statistical tools used to analyze randomized controlled trials
Introduction

Definition of a Randomized Controlled Trial (RCT)

- A study where participants meeting eligibility requirements are randomized into an experimental group or a control group. The experimental treatment and its alternative are clearly defined and the protocols for implementation are tightly managed by the researcher. (ADA terms on EAL)
Randomized Controlled Trial Setup

- **Group Assignment**
  - Experimental Group
  - Control Group

- **Measurement or Observation**
  - Pre-test
  - No Intervention or Placebo

- **Application of Independent Variable**
  - Experimental Intervention

- **Measurement or Observation**
  - Post-test

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Distinguishing Features

• Random assignment to groups
  – A homogenous group of research participants eligible for the experimental treatment(s) or condition(s) is identified
  – Each one of them is, by chance, assigned to one of the treatment conditions or to a control group

• Prospective design

• Can establish cause and effect
Study Variables

• Independent Variable
  – the exposure (the presumed causal factor) is the factor that is thought to influence the dependent variable and that is manipulated in an experimental design (Monsen and Van Horn).

• Dependent Variable
  – the outcome variable of interest (Monsen and Van Horn)

Practice Example

What is the independent or dependent variable?

Example: A researcher is studying the effect of sleep on aggression, thinking that less sleep will lead to more aggression. She has some people sleep 6 hours per night, some people sleep 3 hours per night, and some people sleep as much as they want. She then monitors aggressive behavior during basketball games among participants.
Aggression (or aggressive behavior) is the **dependent variable**. The researcher is studying the effect of sleep on aggression. The level of aggression *depends* on the amount of sleep.

The **independent variable** is the amount of sleep. Remember that the independent variable is the one that the researcher manipulates, in this case, letting some people sleep only 3 hours, others 6 hours, and others, as much as they want.
Questions Answered by an RCT

- **Efficacy**
  - Does the treatment/intervention work?

- **What is the magnitude of effect (on the dependent variable)?**
  - How much did the treatment group(s) differ from the control group in the desired outcome?

- **What proportion benefit?**
  - Did the treatment or control group benefit from the intervention?

- **Which approach is better?**
  - Was it better that the treatment group received the intervention or not?
External Validity

• Definition
  – extent to which study findings can be generalized beyond the sample used in the study (Burns and Grove)

• How to ensure
  – Select a group (sample) that is representative of the population being studied (reference population)
  – Blind all individuals regarding who is in the control or treatment group, including participants, investigators, and data collectors
    • Blinding is possible for some treatments (e.g. drug trial) but not others (e.g. two different nutrition education programs)

Internal Validity

• Definition
  – the extent to which the effects detected in the study are a true reflection of reality, rather than being the result of the effects of extraneous variables (Burns and Grove)

• Maxims for cause-effect or causal relationships
  – The "cause" precedes the "effect" in time (temporal precedence)
  – The "cause" and the "effect" are related (covariation)
  – There are no plausible alternative explanations for the observed covariation

• To ensure internal validity
  – use reliable (reproducible) measures for the independent and dependent variables
  – provide a strong justification for the causal link between the independent and dependent variables

The Validity Questions are Cumulative...

- **Conclusion**: Is there a relationship between cause and effect?
- **Internal**: Is the relationship causal?
- **Construct**: Can we generalize to the constructs?
- **External**: Can we generalize to other persons, places, times?
- **Validity**

Typical Statistics

- Mean and standard deviation
- T-test
- Analysis of variance
- Multivariate analysis (involves observation and analysis of more than one statistical variable at a time)
- Chi square, logistic regression
- Relative risk (risk of an event relative to exposure)
Key Points

• Random assignment into groups
• Can establish cause and effect
• Test efficacy or effectiveness
Resources

Used in the RCT Practice:

• Polley BA, Wing RR, Sims, CJ. Randomized controlled trial (vs. control) to prevent excessive weight gain in pregnant women. *Inter J of Obesity*. 2002;1494-1502.
  
  http://www.nature.com/ijo/journal/v26/n11/full/0802130a.html

Other well-known randomized controlled trials:

  
  http://content.nejm.org/cgi/content/short/344/1/3