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Outcome Measures, Variables and Data Collection

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Objectives

- A few more words about study design…
- Discuss aspects of measurements of outcome and data collection
Study Design

Experimental Group

• the investigational drug
• the “test” treatment or intervention

Control Group

• standard of care; active control
• “gold standard” therapy
• comparator group

To placebo or to not placebo

• what question are you trying to answer?
• ethical issues
Study Design

• Placebo control
  – no standard or accepted treatment or controversy surrounding validity of standard treatment
  – “add on” or “supplemental” therapy
  – “no better than placebo”

• Ethics
  – cardiovascular dz, infectious disease, cancer, diabetes
Variables, Variables, Variables

- Your study design has to have a *primary* outcome that can be *measured*
  - patients will feel better
  - to test whether the drug is effective
  - keep your aims and hypothesis in mind

- Perception vs. measurement

- For example, “effectiveness” of a health program or intervention
  - morbidity, mortality, utilization, incidences of illnesses, costs, quality of life
End Point Variable(s)

- Describe the variable(s) you will use as your primary end point
  - e.g., change in blood pressure
- Describe HOW you will measure your primary end point
- Think about what type of data will you generate
  - nominal
  - ordinal
  - interval scale
  - ratio scale
- READ THIS!
  - Understanding Statistics: An approach for the clinician
Nominal Data

• Enables the classification of individuals, objects or responses based on a common property or characteristic (these are categorical data)
  – gender
  – Democrat or Republican
  – religion (e.g., Christian, Muslim, Hindu)
  – ethnicity (e.g., white, black, Hispanic)

Ordinal Data

• Ranks data based on a scale
  – income (above average, average, below average)
  – attitude- Likert scale (strongly agree, agree, neutral, disagree, strongly disagree)
    • symptom severity
    • “subjective” or “perception” end points
    • when using a “referenced” or validated tool include it in your proposal as an appendix

Interval Scale

- Has a unit of measure with an arbitrary starting point and ending point
  - temperature
  - blood pressure
  - cholesterol

Ratio Scale

• Numerical data
  – discrete- how many cars do you have?
  – continuous- how tall are you?
  – can have a “zero” point
  – height, weight, age
Controlling for Confounding Variables

Smoking
Independent Variable
Assumed Cause

Lung Cancer
Dependent Variable
Assumed Effect

- age
- family history
- genetic disposition
- lifestyle variables

Confounding Variables

- Methods for controlling for confounding variables
  - randomization
  - age or gender matching
  - identification and measurements of known confounding variables (e.g., secondary end points)
  - unanticipated

- Timing and frequency of data collection
Data Collection

• The study’s primary end point
  – define it
    • it should be very clear from reading your proposal what your study’s primary end point is
  – describe when and how you’ll collect it in relationship to your intervention
Develop Your Study Design

• should effectively test your hypothesis and should be detailed enough so that the variables that will be measured and how data will be collected are apparent

• the study population should be described and inclusion/exclusion criteria presented (if applicable)

• the test groups (e.g., treatment and placebo) should be mentioned

• the study’s primary end point (variable) should be clearly stated

• THESE ELEMENTS NEED TO BE CLEAR!

• READ! articles posted in “other resources”

• RUBRIC, RUBRIC, RUBRIC!
Statistical Plan

• Your study design will direct the development of your statistical plan
  – the TYPE of data (e.g., ordinal) your study will generate will direct your statistical plan
  – Dr. Michael Dorsch will give the lecture on developing a statistical plan (2/24/11)
  – Assignment #3: study design, statistical plan, human subjects/vertebrate animals change in due date
    • now 3/14/11
    • updated schedule posted