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Pharmacy 476
Selecting a Study Design

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Objectives

• Discuss the process of selecting a study design
• Address project-specific questions about study design
Study Designs

Prospective

Randomized, placebo-controlled

Retrospective

Observational, case-controlled

Double-blind, cross-over

Double-dummy, three-way
Most Common Study Designs

Number of Contacts with the Study Population

- Three different perspectives to consider:
  - number of contacts with the study population
  - duration of study or the length of the study period
  - the type of investigation

Study Design
Based on the Number of Contacts

• Study design based on the number of contacts falls into one of three groups:
  – cross-sectional
  – before-and-after
  – longitudinal

Study Design
Cross-sectional Study Design

• Takes a “snap-shot” in time
  – takes a “cross-section” of a population
  – can be prospective or retrospective

• Best suited for:
  – finding out the prevalence of a disease or phenomenon

• Is simple in design
  – identify the study population
  – select a sample
  – acquire data

Cross-sectional Study Design

• Examples
  – the attitude of the elderly towards pharmacists
  – the socioeconomic-demographic characteristics of teenagers with STDs
  – the relationship between the home environment and the incidence of asthma in children

• These studies involve only one contact with the study population

Study Design
Before-and-After Study Design

• Measures change
  – It’s the most appropriate design for measuring the impact or effectiveness of an intervention or program:
    • can be two-cross sectional studies
    • can be prospective or retrospective
    • “impact” or “effect” on a study population

Before-and-After Study Design

• Examples
  – The impact of specialty board certification on the quality of pharmacy services
  – The effectiveness of pharmacy-based management of hypertension
  – The impact of an antibiotic restriction program on hospital drug costs
Before-and-After Study Design

• Advantages
  – measures change and/or impact/effectiveness

• Disadvantages (depends on type of study)
  – requires the collection of two (rather than one) data sets from the same study population
  – time between measures may result in attrition of study population or can skew its demographics (e.g., pediatrics)
  – measures total change- you can’t ascertain whether other variables contribute to the change in your measure
  – “intervention” may condition study population
  – study population attitudes can be influenced over time

Study Design
Longitudinal Study Design

- Used to study patterns of change over time
  - to study the proportion of people adopting a program in relation to time
  - you can collect data on a continuing basis and measure trends

- Study population is sampled numerous times
  - a series of repetitive cross-sectional studies

Longitudinal Study Design

• Advantages
  – allows for the measure of changes in patterns over time on a regular or continuous basis
    • effect of propranolol on blood pressure using a continuous blood pressure monitoring device

• Disadvantages
  – conditioning effect

Most Common Study Designs

Duration of the Study

• Three different perspectives to consider:
  – number of contacts with the study population
  – duration of study or study period
  – the type of investigation

Study Design
Based on Study Length

- Study design based on the duration of the study or the length of the study period falls into one of three groups:
  - retrospective
  - prospective
  - retrospective-prospective

Study Design
Retrospective

• Retrospective studies investigate a phenomenon or problem that has happened in the past
  – fact-finding, preliminary, hypothesis-generating
  – important event that occurred in the past

Prospective studies refer to the future to determine the impact or an effect of an intervention on a future event.
Study Design
Retrospective-Prospective

- These studies use past trends to study future events
  - measurement of an intervention without having a control group (e.g., use of historical controls)
  - before-and-after studies can be retrospective-prospective
    - part of the data are collected before an intervention from existing records then the same group is studied following an intervention

Randomization

- Experimental studies can be further classified on whether the study population is randomly assigned to different treatment groups
  - the intent is that potentially confounding variables will be equally distributed between the groups
  - can be used in any experimental design
Study Design

Experimental

• before-and-after design
Study Design
Experimental

- Addition of inclusion/exclusion criteria, randomization and control groups
Study Design

Experimental

- Can have more than one experimental and/or control groups
  - may also have more than one treatment or intervention (arms)
  - this will be dictated by your hypothesis and your specific aims
Study Design
Experimental

- Control groups can consist of an active control, a placebo control or both
Study Design

Experimental

- Other designs
  - Cross-over

```
Group A
  control treatment
  active treatment
  control treatment
Group B
  active treatment
  control treatment
Group A
```

“wash-out”

Time

Kathleen A. Stringer
Study Design

- **Case control study**: characteristics of patients with a condition are compared with those without. What’s the cause?
- **Cohort study**: group of patients with a specific disease or characteristic are followed over a period of time to detect complications or new events. Can be retrospective or prospective.
- **Cross-sectional**: presence or absence of exposure to possible risk factor measured at one point in time. Obtains prevalence.
Study Design

• Blinding
  – limits bias in the conduct of research and the interpretation of data
  – bias influences recruitment, enrollment, group allocation, decision-making, data analysis and assessment
  – Types: double-blind, single-blind

• Unblinded or open label
Study Design
The Devil’s in the Details

• Identify the study design you will use to test your hypothesis and achieve your aims

• Expand to include more details
  – inclusion/exclusion criteria (avoid redundancy)
  – treatment regimens or interventions
    • what exactly are you going to test?
Study Design

• Define your patient population
  – who (gender, age, ethnicity etc)
  – disease state (specific criteria)
  – setting (inpatient, outpatient)

• These parameters are what you’ll use to define your inclusion/exclusion criteria

• You need to provide enough detail so your reviewer knows what you plan to do
Study Design

• Method details
  – describe drug regimen (if applicable)
    • what?
    • how much (and frequency)?
    • how long?
  – treatment arms
  – specifics of intervention
Measurements

• On Monday we’ll talk about outcomes, variables and measurements
  – these are also aspects of your study design

• Individual assignment #2
  – due TOMORROW via Ctools assignments