M6728

Research Designs

Goals

- Match appropriate research designs to the study purpose and questions
- Differentiate between experimental and non-experimental studies
- Discuss epidemiologic designs
- Evaluate designs of studies in literature
- Identify types of study validity and potential threats to validity

Depression Scores

<table>
<thead>
<tr>
<th>Baseline</th>
<th>Old Drug</th>
<th>New Drug</th>
</tr>
</thead>
<tbody>
<tr>
<td>23.9</td>
<td>12.8</td>
<td>13.4</td>
</tr>
<tr>
<td>26.0</td>
<td>13.4</td>
<td>13.0</td>
</tr>
<tr>
<td>28.1</td>
<td>20.3</td>
<td>19.4</td>
</tr>
</tbody>
</table>
**Depression scores again**

<table>
<thead>
<tr>
<th></th>
<th>Baseline</th>
<th>Old Drug</th>
<th>New Drug</th>
<th>Placebo</th>
</tr>
</thead>
<tbody>
<tr>
<td>Depression</td>
<td>23.9</td>
<td>12.8</td>
<td>13.4</td>
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<tr>
<td>Depression</td>
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<td>28.1</td>
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<td>19.4</td>
<td>18.9</td>
</tr>
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**What is an experiment?**

- **Manipulation:** investigator intervenes or changes something
- **Control:** a comparison group without the intervention
- **Randomization:** each subject has an equal chance of receiving the intervention

**Example: Antiseptic Handwashing and Infections**

- **Hypothesis:** There is no difference in nosocomial infection rates among patients receiving care from staff who use an antiseptic soap or a non-antimicrobial soap.
The experimental elements....

- Manipulation: type of soap
- How and what to compare?
- What to randomize?
  - Individual subjects to one soap or another?
  - Order of soap?
  - Clinical units?

Quasi-Experimental Designs

An intervention, but no randomization

Pre-Post Test Control Group

<table>
<thead>
<tr>
<th></th>
<th>X</th>
<th></th>
</tr>
</thead>
<tbody>
<tr>
<td>0</td>
<td></td>
<td>0</td>
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</tbody>
</table>
Repeated Treatment

Subjects act as their own controls
\[0 \times 0 \times 0 \times 0 \times 0 \times 0\]

Crossover Design

Same subjects, different interventions
\[0 \times a \times 0 \times b \times 0 \times a \times 0 \times b\]

Crossover Study Examples

- **Subjects:** Insulin-dependent diabetics
- **Treatment:** Insulin injection into arm, leg, abdomen
- **Outcome:** Insulin absorption, glucose levels

- **Subjects:** Non-coffee drinkers
- **Treatment:** Caffeine or placebo
- **Outcome:** Plasma renin, catecholamine, cardiovascular function
Post-test Only Control Group

x₀
- ū₀

One Group Pretest-Posttest

₀ → x → ₀

Post-test Only

Non equivalent groups

x → ₀
- → ₀
Pros

Experiment | Quasi-Experiment

- More controls
- More internal validity
- Fewer rival hypotheses
- More practical
- More feasible
- More generalizable

Why not always an experiment?

- Can’t manipulate certain variables (sex, age, race, profession)
- Unethical (e.g. can’t have a control group with no handwashing)
- Impractical or undesirable (insufficient time, resources, cooperation, inability to randomize)

Non-Experimental Designs

No intervention
Sometimes in Health services research it is called a “natural experiment”
ICUWC
Classifying Non-Experimental Designs

- By time: retrospective, cross-sectional, prospective
- By method: survey, observational, historical, case study, qualitative
- By purpose: description, correlation, prediction, evaluation, methodologic

Descriptive Research by Timing

Retrospective: Effect → Cause
Prospective: Cause → Effect

Retrospective (case-control)

- Start with an event (e.g. a disease) and look back to see what factors may have caused the event or disease
- Frequency of factor is compared among those who are diseased (cases) and those who are similar but don't have the disease (controls)
- RR (measured by odds ratio) must be higher in cases than controls
Use a retrospective approach when...
- The suspected cause (disease) is rare;
- Exposure is common among diseased;
- An event has already occurred (e.g. an outbreak investigation)

Case-Control Pros and Cons
- Relatively quick, easy, economical
- Difficult to make causal inferences (e.g. Which came first—the exposure or disease?)
- Finding appropriate controls may be difficult

Sources of Cases and Controls

Cases
- All cases diagnosed in a community
- All cases in a single hospital
- All cases from one or more hospitals

Controls
- Sample of gen. pop. in same community
- Pts. from same hospital without the disease
- Persons resident in same block or neighborhood as cases
Prospective (cohort) Studies

- Start with a condition (e.g. exposure) and look forward
- Frequency of the outcome (e.g. a disease) is compared between those with and without the exposure
- RR must be higher in those exposed

Use A Prospective Approach When....

- Suspected exposure (cause) is not common, but effect (disease) of interest is frequent among those exposed;
- Time between exposure and disease is short;
- Attrition can be minimized
- Investigator has a long life expectancy

Cohort Study Pros and Cons

- Better able to establish causality;
- Expensive, time consuming, difficult to maintain follow up;
- Selection of non-exposed comparison group difficult
What’s your choice? An association between....

- Smoking and peptic ulcer disease?
- Radiation exposure and breast cancer?
- Cholesterol and heart disease?
- Home health care and patient’s functional status?
- Hepatitis and needlesticks?

Survey Research

Data gathered from a portion of a population to examine characteristics, opinion, intentions (e.g. census, vital statistics)

Pros and Cons of Surveys

- Flexible, allows access to many subjects;
- Data may be superficial;
- Low return rate
**Historical Research**

Systematic collection and critical evaluation of past data

**Types of Historical Data**

- Primary sources: original documents, first hand information, witnesses
- Second sources: textbooks, references

**One-shot Case**

$X + 0$
To evaluate historical data:

- External criticism: are data authentic and genuine?
- Internal criticism: is the content of the data accurate (i.e. was the writer unbiased) and worthwhile?

Methodological Research

To develop and test tools or techniques

Study validity...

- Measures the accuracy of a claim
- So important (the crux of a study's value), but so difficult to assess
Questions to Ask (in EBP is this study valid?)

- Is there a relationship between the variables (statistical conclusion validity)?
- Is it plausible that the relationship is causal (internal validity)?
- If there appears to be a causal relationship, are the cause-and-effect constructs measured accurately (construct validity)?

Internal Validity

Extent to which the relationship detected found is truth

Threat to Internal Validity

- HISTORY
  An event not related to the planned study but occurring at the same time that affects study results
**Statistical Conclusion Validity**

Whether conclusions about relationships and differences drawn from analyses are an accurate reflection of reality (i.e., did not occur by chance)

**Threat to Internal Validity**

- **MATURATION**
  Changes among subjects (e.g. growing older, wiser, more experienced) during the study in ways that affect the study results

**Threats to Internal Validity**

- **TESTING**
  Effect being measured is due to previous testing
- **INSTRUMENTATION**
  Effect due to measurement instrument rather than treatment (e.g. more experienced observers, change in instrument)
Threats to Internal Validity

- MORTALITY/DROPOUT
  Those who drop out of a study differ from those who stay in, or drop out occurs differentially in experimental and control groups

Questions to Ask (In EBP, is this study applicable?)

- How generalizable is this relationship to other settings, times, persons (external validity)?

External Validity

Extent to which findings can be generalized beyond the sample
Threats to External Validity

- Hawthorne or novelty effects
- Interaction of treatment and history, setting or selection
- Investigator effects
- Measurement effects