Study Design (part 2)

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Example

- Suppose we want to know: "Is arthroscopic surgery is effective in treating arthritis of the knee?" Describe both an *observational study* and a *randomized experiment* that you could conduct to answer this question. Be sure to address the following during your discussion:

1. How costly will it be for the researchers to collect data with each design?
2. Are there any feasibility problems or ethical issues with each design?

Example Answers

- An observational study might use medical records to find patients with knee arthritis and identify whether or not they ever received surgery. It then might compare various measures of recovery (recurrent visits, etc.) among those who had or didn't have surgery.
- A randomized experiment would take a sample of patients with arthritis and randomly assign half to have surgery and the other half to not have surgery. It then might compare various measures of recovery.

1. The observational study costs almost nothing, while the randomized experiment likely will cost quite a bit
2. The observational study doesn't present any ethical barriers (assuming sufficient data privacy), but withholding knee surgery seems like it could problematic

Sham Knee Surgery

In the 1990s a study was conducted in 10 men with arthritic knees that were scheduled for surgery. They were all treated identically expect for one key distinction: only half of them actually got surgery! Once each subject was in the operating room and anesthetized, the surgeon looked at a randomly generated code indicating whether he should do the full surgery or just make three small incisions in the knee and stitch up the patient to leave a scar. All patients received the same post-operative care, rehabilitation, and were later evaluated by staff who didn't know whether they had actually received the surgery or not. The result? Both the sham knee surgery and the real knee surgery showed indistinguishable levels of improvement

Source: https://www.nytimes.com/2000/01/09/magazine/the-placebo-prescription.html

Control Groups, Placebos, and Blinding

The Sham Knee Surgery example illustrates several important aspects of a well-designed experiment that we've yet to discuss:

- **Control Group** - Some patients were randomly assigned not to receive the knee surgery, providing a comparison group that is, on average, balanced with surgery group in all baseline characteristics
- **Placebo** - Patients in the control group received a fake surgery
- **Blinding** - Using a placebo is not helpful if patients know which group they're in. Similarly, the staff interacting with the patients might treat them differently if they knew the patient's group
  - **Single-blind** - the participants don't know the treatment assignments
  - **Double-blind** - the participants *and* everyone interacting with the participants don't know the treatment assignments

Control Groups, Placebos, and Blinding

- The goal of each of these design elements is to prevent biasing the measurement of the outcome variable in a particular direction

Control Groups, Placebos, and Blinding

- The goal of each of these design elements is to prevent biasing the measurement of the outcome variable in a particular direction
- Other types of studies are susceptible to other types of biases, which we should also carefully consider

1. Social Desirability Bias - Respondents tend to answer questions in ways that portray themselves in a positive light Link
2. Habituation Bias - Respondents tend to provide similar answers for similarly worded or structured questions (the brain going on autopilot) Link
3. Leading Questions - The wording of a question impacts how people respond, great examples in the textbook
4. Cultural Bias - Questions are often to be constructed with one's own culture in mind, they might not even make sense to people from other cultures.

Discussion - Can Randomization Fail?

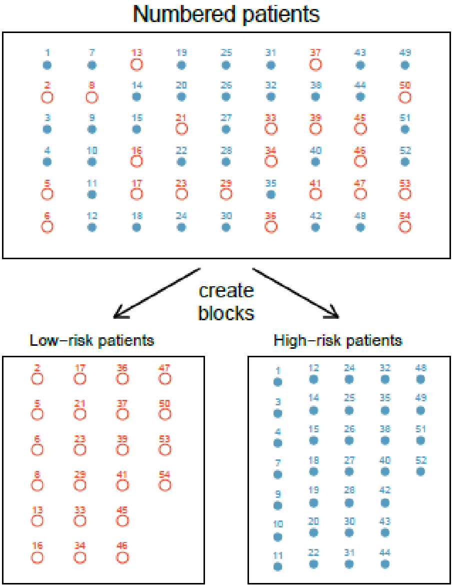
- A University of Iowa researcher was conducting an experiment on lab monkeys
- Lab monkeys are expensive, so his experiment had $n = 8$
- Having taken a statistics course, he randomly assigned treatment/control groups
- After conducting the experiment and seeing surprising results, the researcher recognizes that the 4 monkeys in the control group were also the oldest 4 monkeys
- The researcher knew that the age of the monkey had an important on the outcome variable, but he expected randomization to handle that

Should he report his results? What could he have done differently?

Can Randomization Fail?

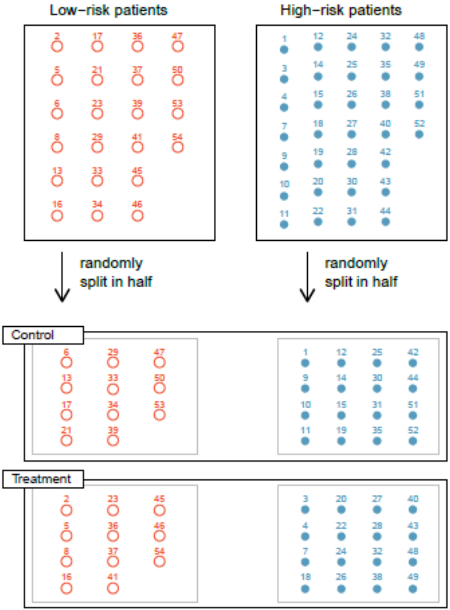
- Randomization is not guaranteed to properly balance the treatment and control groups unless the sample size is relatively large
- At smaller sample sizes, strategies such as **blocking** can be used
  - In this design, cases are first split using a **blocking variable**, then random assignment is done within each block
  - This ensures the blocking variable is balanced in each group

Blocking



Numbered patients
create
blocks
Low−risk patients
High−risk patients

Blocking



Low–risk patients
High–risk patients
randomly
split in half
randomly
split in half
Control
Treatment

Study Design and Statistical Inference

- The overarching goal of a statistician is to *rule out* as many possible explanations for an observed association as possible

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- The overarching goal of a statistician is to *rule out* as many possible explanations for an observed association as possible
- So far we've considered the following design-related explanations, as well as methods for addressing for them
  - Sampling bias - Simple random sampling
  - Confounding variables - Random assignment of the explanatory variable, or stratification
  - Other biases - Using placebo, double-blinding, proper instruments, etc.

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  - Random chance
  - The association is real

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  - Random chance
  - The association is real
- Up next we'll work on formalizing the framework statisticians use to decide between these two remaining explanations