# Mathematics 231

Lecture 13 Liam O'Brien

#### Announcements

Reading

■ Today M&M 3.1 178-191

■ Next class M&M 3.2 197-207

# **Topics**

- Collecting data
  - Sampling
  - Observational studies
  - Experiments

#### What is Statistics?

Collecting Data

Describing Data

Drawing Conclusions from Data

# Collecting Data

- Production of data requires answers to the following questions:
  - Who or what is the object of the study units?
  - What variables should be measured?
  - How should we select the individuals/groups/countries?

# Collecting Data

- Statistical designs for producing data usually rely on either sampling or experiments.
- **Sampling:** Basic idea of sampling is to study a "part" to gain information about the "whole."
- Examples:
  - U.S. opinion polls often use only 1000-1500 people.
  - U.S. unemployment surveys use approximately 50,000 households.

# Sampling

- Reasons for sampling:
  - Cost: Too expensive to "sample" everyone.
  - Timeliness: Too time-consuming.
- However, to ensure sound conclusions the sampling design is very, very important.

#### Observational Study vs. Experiment

- A sample survey collects information on a population, leaving it undisturbed by the process.
- Sample surveys are a type of observational study.
- In an observational study there is no attempt to influence the responses.

### Experiments

- An experiment imposes some "treatment" on the individuals in order to observe their responses.
- An experiment allows us to control or eliminate lurking variables.
- In principle, experiments are the "gold standard" of evidence to support "causation."
- Experiments may not always be ethical/practical.

## Example: Study of Welfare

- In a large observational study, children on welfare were found to have behavioral problems later in life.
- However, the effect of being on welfare on children's behavior is confounded with the socioeconomic status of the families that need welfare.
- In principle, an experiment can control or eliminate these effects.
- Is this ethical/practical?

#### Observational Study vs. Experiment

- In an observational study there is no attempt to influence the responses.
- An experiment imposes some "treatment" on individuals in order to observe their responses.
- An experiment allows us to control or eliminate lurking variables.

### Example: Pheromones

- **Pheromones**: Chemicals that regulate behavior in many animals.
- Pheromones secreted by one animal affect behavior of another animal (e.g., mating, finding food, escaping enemies).
- Is there any evidence of human pheromones?

### Example: Human Pheromones

■ Martha McClintock — Wellesley Class of 1969



#### Example: Human Pheromones

- McClintock 1971 study.
- McClintock conducted a study of 135 women in her dorm; wrote up the results as senior thesis.

Later published in *Nature*, considered the first scientific evidence of functioning of human pheromones.

# McClintock 1971 Study

- Recorded menstrual period onset dates of students in Wellesley dorm in October.
- By March, found close friends and roommates had onset dates that were closer in time.
- Evidence for pheromones in humans?
- Confounding variables?

## McClintock 1998 Experiment

- Researchers gathered samples from 9 women by placing pads under their armpits for 8 hours, taken during 2 different phases of the menstrual cycle.
- Pads frozen in glass vials, then swiped under the noses of 20 women.
- Depending on when in the cycle the samples were taken, they could shorten or lengthen cycles in other women.

# Design of Experiments

- Terminology
- The units are the subjects of the study.
- A specific experimental condition is called a treatment.
- The distinction between response and explanatory variable is usually clear.
- Explanatory variables are often called factors and specific treatments are called levels.
- Note: Specific **treatments** can be formed by combinations of different factors.

#### Example: Physicians Health Study

■ Units: 21,996 male physicians

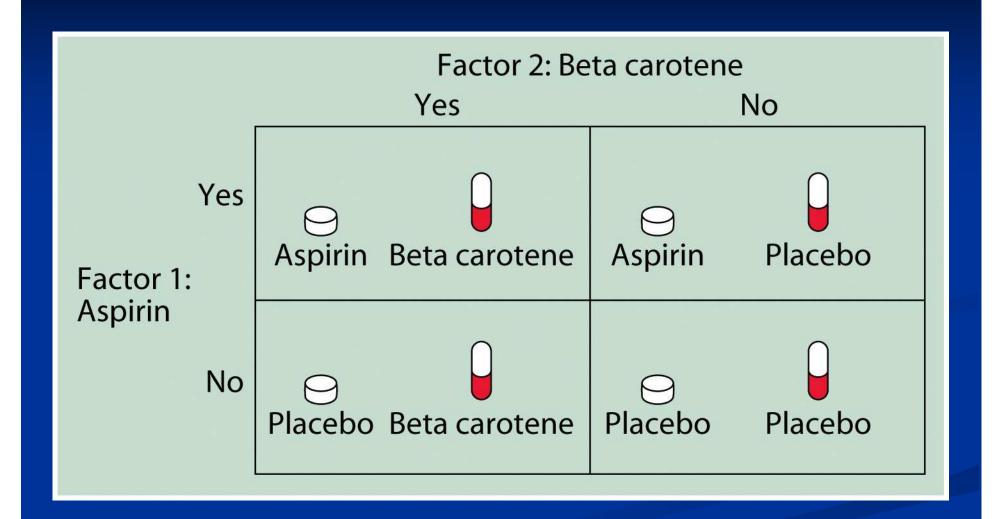
Factors: 2 factors

Aspirin vs. placebo

Beta carotene vs. placebo

→ 4 (treatment) levels

■ Response: Heart disease/cancers



# Design of Experiments

- Single Treatment Experiment
- What if we assign all units to receive the treatment? What can we say about the response?
- We need a **control group** to compare to.
- Control is the 1<sup>st</sup> principle of experimental design.

## Control Group

- **Control group** differs from the treatment group only in terms of the treatment assignment.
- It allows us to eliminate or reduce the effects of experimental arrangements, subject selection, and placebo effects.
- If these are not controlled, the result is **bias** (i.e., study systematically favors certain results.

#### Placebo Effects

- Placebo effect is the measurable, observable, or felt improvement not attributable to treatment.
- Example: Patients suffering pain after wisdom tooth extraction got just as much relief from a fake application of ultrasound as from a real one (provided both the doctor and patient thought the machine was on).

### Assignment to Treatments

- The 2<sup>nd</sup> principle of experimental design concerns assignment to treatments.
- How to make assignments in a way that's fair to all treatments.
- **Matching**: We can match someone who receives one treatment to someone who receives another based on important factors (e.g., age, gender, smoking status).
- **Randomization**: Use chance to decide.

#### Randomization

- Randomization produces treatment groups that are similar in all respects except treatment received.
- So differences in the response must be due to the treatment differences or to chance.
- The effects of chance should average out with a large enough sample size.
- A treatment effect so large that it would rarely occur by chance is said to be "statistically significant."

# Principles of Experimental Design

- 1. Control effects of lurking variables by comparing two or more treatments.
- 2. Use randomization to assign units to treatments.
- 3. Replicate each treatment on many units to reduce chance variation.

# Blinding

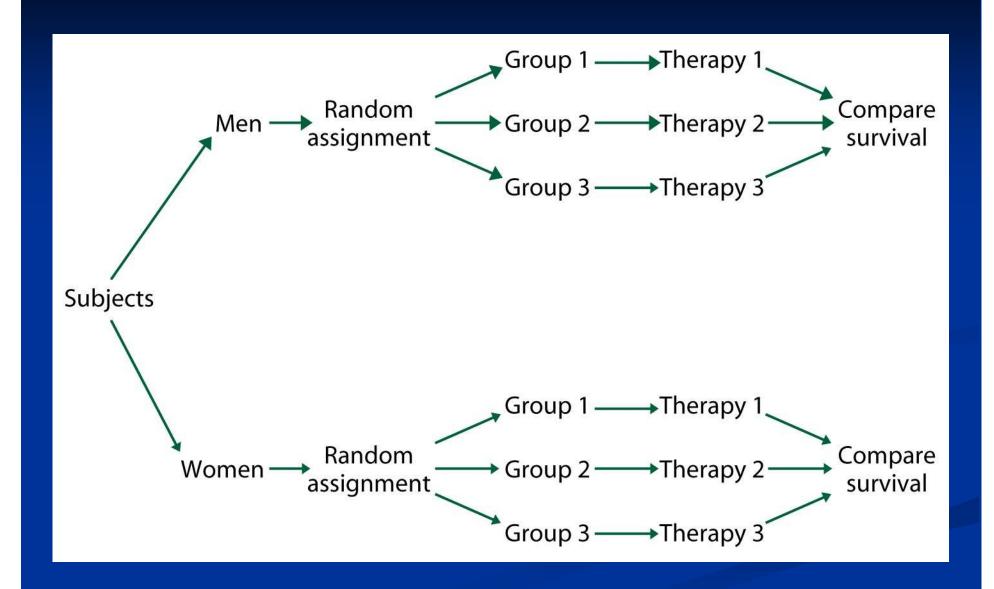
- Blinding: Comparison of treatments can be distorted if subjects, persons administering or evaluating treatment know which treatment if being allocated.
- Blinding avoids many sources of unconscious bias.
- **Double-blind**: Neither subjects nor experimenters know which treatments have been received.
- Single Blind: Subjects do not know which treatment they have received.

# Blocking

- **Blocking**: A block is a group of units known to be similar in some way that is thought likely to influence the response variable.
- In a "randomized block design," randomization is carried out separately within each block.
- Example; Matched-pairs design
  - Blocks consisting of two units matched as closely as possible, e.g., one active eye drop in one eye and a placebo in the other.

# Block Design

- Consider an experiment designed to compare three chemotherapies for a type of cancer that progresses differently in males and females.
- Units: 60 subjects (30 males, 30 females)
- **Factor**: Therapy (3 levels)
- Block: Gender (blocks consist of males and females)
- Response: Survival



#### Choice of Blocks

- Blocks should be chosen on the basis of the most important (known) unavoidable source of variation among experimental units.
- Randomization then averages out the remaining sources of variability to allow unbiased estimation of treatment effects.
- Blocks allow greater precision, because a source of systematic variation is removed (reduced variability) from the experimental comparison.