Stat 13, Intro. to Statistical Methods for the Life and Health Sciences.

- 1. Some good hw problems from the book.
- 2. Blinding.
- 3. Portacaval shunt example.
- 4. Coverage, adherer bias, and clofibrate example.
- 5. More about confounding factors.
- 6. Lefties example.

Finish reading chapter 4. http://www.stat.ucla.edu/~frederic/13/W23 . HW2 is due Fri Feb10 at 2pm.

HW3 is due Wed Feb22, 2pm, to statgrader@stat.ucla.edu or statgrader2@stat.ucla.edu. Midterm is Fri Feb24 in class.

HW3 is 4.CE.10, 5.3.28, 6.1.17, and 6.3.14.

- In 5.3.28d, use the theory-based formula. You do not need to use an applet.
- 4.CE.10 starts out "Studies have shown that children in the U.S. who have been spanked have a significantly lower IQ score on average...."
- 5.3.28 starts out "Recall the data from the Physicians' Health Study: Of the 11,034 physicians who took the placebo ...."
- 6.1.17 starts out "The graph below displays the distribution of word lengths ...."
- 6.3.14 starts out "In an article titled 'Unilateral Nostril Breathing Influences Lateralized Cognitive Performance' that appeared ...."

1. Some good hw problems from the book.

1.2.18, 1.2.19, 1.2.20, 1.3.17, 1.5.18, 2.1.38, 2.2.6, 2.2.24, 2.3.3, 2.3.25, 3.2.11, 3.2.12, 3.3.8, 3.3.19, 3.3.22, 3.5.23, 4.1.14, 4.1.18, 5.2.2, 5.2.10, 5.2.24, 5.3.11, 5.3.21, 5.3.24, 6.2.23, 6.3.1, 6.3.12, 6.3.22, 6.3.23.

## 2. Blinding.

Even in experiments, the treatment and control groups can be different in ways other than the explanatory variable. This is especially true when the response variable is somewhat subjective. Pain is an example. One study found that 1/4 of patients suffering from post-operative pain, when

given a placebo (just a pill of sugar and water) claimed they experienced "significant prompt pain relief".

## Blinding.

People might not be able to judge their own levels of pain very well, and may be influenced by the belief that they have taken an effective treatment.

Thus in an experiment with such a response variable, researchers should ensure the subject does not know whether he or she received the treatment or the control. This is called blinding.

In a *double-blind* experiment, neither the subject nor the researcher recording the response variable knows the level of the explanatory variable for each subject, i.e. treatment or control.

## 3. Portacaval shunt example.

The following example shows the importance of doing a randomized controlled experiment.

The portacaval shunt is a medical procedure aimed at curbing bleeding to death in patients with cirrhosis of the liver.

The following table summarizes 51 studies on the portacaval shunt. The poorly designed studies were very enthusiastic about the surgery, while the carefully designed studies prove that the surgery is largely ineffective.

	Degree of enthusiasm			
Design	High Moderate None			
No controls	24	7	1	
Controls, but not randomized	10	3	2	
Randomized controlled	0	1	3	

## Portacaval shunt example.

Why did the poorly designed studies come to the wrong conclusion?

A likely explanation is that in the studies where patients were not randomly assigned to the treatment or control group, by and large the healthier patients were given the surgery.

This alone could explain why the treatment group outlived the control group in these studies.

	Degree of enthusiasm			
Design	High Moderate None			
No controls	24	7	1	
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Randomized controlled	0	1	3	

# 4. Coverage, adherer bias and Clofibrate example.

Surveys are observational.

- Coverage is a common issue. Coverage is the extent to which the people you sampled from represent the overall population.
   A survey at a fancy research hospital in a wealthy neighborhood may yield patients with higher incomes, higher education, etc.
- Non-response bias is another common problem. Poor coverage means the people getting the survey do not represent the general population. Non-response bias means that out of the people you gave the survey to, the people actually filling it out and submitting it are different from the people who did not.
- Same exact issues in web surveys.

# Coverage, adherer bias, and Clofibrate example.

Non-response bias is similar to adherer bias, in experiments.

A drug called clofibrate was tested on 3,892 middle-aged men with heart trouble. It was supposed to prevent heart attacks.

1,103 assigned at random to take clofibrate,

2,789 to placebo (lactose) group.

Subjects were followed for 5 years.

Is this an experiment or an observational study?

Clofibrate patients who died during followup

adherers 15%

non-adherers 25%

total 20%

# Coverage, adherer bias, and Clofibrate example.

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1,103 assigned at random to take clofibrate,

2,789 to placebo (lactose) group.

Subjects were followed for 5 years.

Is this an experiment or an observational study?

It is an experiment. Does Clofibrate work?

Clofibrate patients who died during followup

adherers 15%

non-adherers **25**%

total 20%

adherers 15%

non-adherers 25%

total 20%

\_\_\_\_\_

#### Placebo

adherers 15%

nonadherers 28%

total 21%

Those who took clofibrate did much better than those who didn't keep taking clofibrate. Does this mean clofibrate works?

adherers 15%

non-adherers 25%

total 20%

\_\_\_\_\_

#### Placebo

adherers 15%

nonadherers 28%

total 21%

Those who adhered to placebo also did much better than those who stopped adhering.

adherers 15%

non-adherers 25%

total **20**%

\_\_\_\_\_

#### Placebo

adherers 15%

nonadherers 28%

total **21**%

All in all there was little difference between the two groups.

adherers 15%

non-adherers 25%

total **20**%

\_\_\_\_\_

### Placebo

adherers 15%

nonadherers 28%

total **21**%

Adherers did better than non-adherers, not because of clofibrate, but because they were healthier in general. Why?

adherers 15%

non-adherers 25%

total **20**%

\_\_\_\_\_

#### Placebo

adherers 15%

nonadherers 28%

total **21**%

Adherers did better than non-adherers, not because of clofibrate, but because they were healthier in general. Why?

- adherers are the type to engage in healthier behavior.
- sick patients are less likely to adhere.

## 5. More about confounding factors.

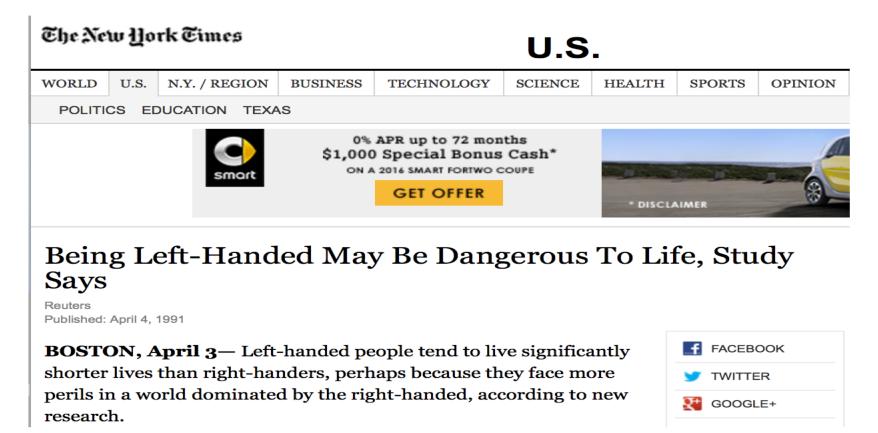
- By a confounding factor, we mean an alternative explanation that could explain the apparent relationship between the two variables, even if they are not causally related. Typically this is done by finding another difference between the treatment and control group. For instance, different studies have examined smokers and non-smokers and have found that smokers have higher rates of liver cancer. One explanation would be that smoking causes liver cancer. But is there any other, alternative explanation?
- One alternative would be that the smokers tend to drink more alcohol, and it is the alcohol, not the smoking, that causes liver cancer.

## 5. More about confounding factors.

- Another plausible explanation is that the smokers are probably older on average than the non-smokers, and older people are more at risk for all sorts of cancer than younger people.
- Another might be that smokers engage in other unhealthy activities more than non-smokers.
- Note that if one said that "smoking makes you want to drink alcohol which causes liver cancer," that would not be a valid confounding factor, since in that explanation, smoking effective is causally related to liver cancer risk.

- A confounding factor must be plausibly linked to both the explanatory and response variables. So for instance saying "perhaps a higher proportion of the smokers are men" would not be a very convincing confounding factor, unless you have some reason to think gender is strongly linked to liver cancer.
- Another example: left-handedness and age at death.
   Psychologists Diane Halpern and Stanley Coren looked at 1,000 death records of those who died in Southern California in the late 1980s and early 1990s and contacted relatives to see if the deceased were righthanded or lefthanded. They found that the average ages at death of the lefthanded was 66, and for the righthanded it was 75. Their results were published in prestigious scientific journals, Nature and the New England Journal of Medicine.

All sorts of causal conclusions were made about how this shows that the stress of being lefthanded in our righthanded world leads to premature death.

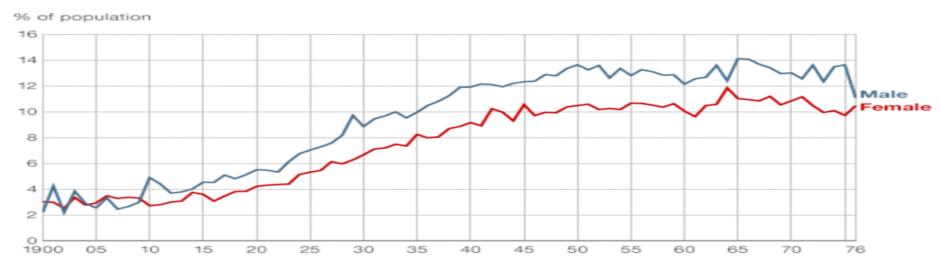


• Is this an observational study or an experiment?

- Is this an observational study or an experiment? It is an observational study.
- Are there plausible confounding factors you can think of?

• A confounding factor is the age of the two populations in general. Lefties in the 1980s were on average younger than righties. Many old lefties were converted to righties at infancy, in the early 20th century, but this practice has subsided. Thus in the 1980s and 1990s, there were relatively few old lefties but many young lefties in the overall population. This alone explains the discrepancy.

#### Left handedness 1900-1976



Source: Chris McManus Right Hand, Left Hand