

# Observational studies

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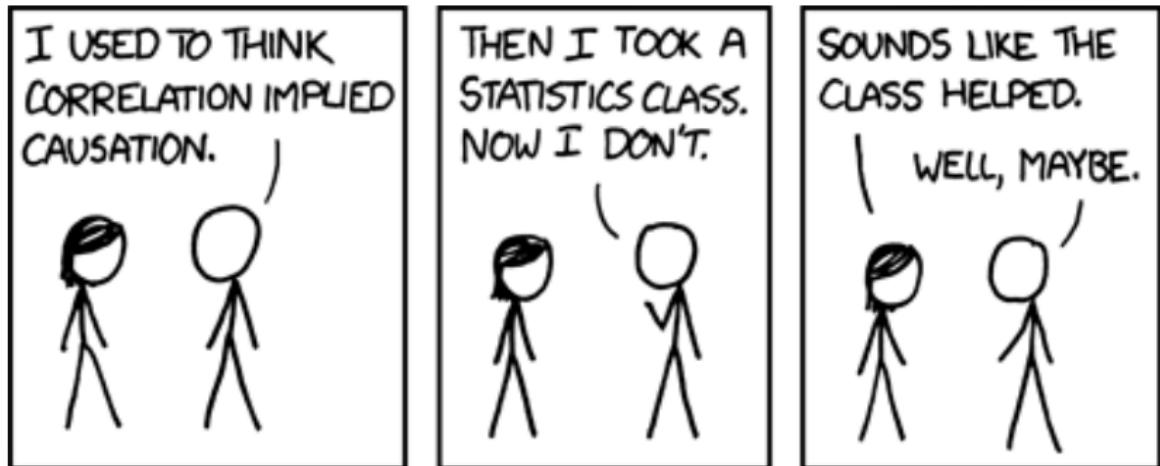
# Observational studies

- We have said that randomized controlled experiments are the gold standard for determining cause-and-effect relationships in human health
- However, such experiments are not always possible, ethical, or affordable
- A much simpler, more passive approach is to simply observe people's decisions and the consequences that seem to result from them, then attempt to link the two
- Such studies are called *observational studies*

# Smoking

- For example, smoking studies are observational — no one is going to take up smoking just to please a researcher
- However, the idea of an exposed group (smokers) and a control group (nonsmokers) is still used, just as it was in controlled experiments
- The essential difference, however, is that the subject assigns themselves to the treatment/control group — the investigators just watch
- Because of this, confounding is possible
- Hundreds of studies have shown that smoking is *associated* with various diseases, but none can prove *causation*

# Correlation and causation



<http://xkcd.com/552/>

# Controlling for confounders

- However, just because confounding is possible in such studies does not mean that investigators are powerless to address it
- Instead, well-conducted observational studies make strong efforts to identify confounders and *control for* their effect
- There are many techniques for doing so; the most direct approach is to make comparisons separately for smaller and more homogeneous groups

## Controlling for confounders (cont'd)

- For example, studying the association between heart disease and smoking could be misleading, because men are more likely to have heart disease and also more likely to smoke
- A solution is to compare heart disease rates separately: compare male smokers to male nonsmokers, and the same for females
- Age is another common confounding factor that epidemiologists are often concerned with controlling for

# The value of observational studies

- Hundreds of carefully controlled and well-conducted studies of smoking have been conducted in the past several decades
- Most people would agree that these studies make a very strong case that smoking is dangerous, and that alerting the public to this danger has saved thousands of lives
- Observational studies are clearly a powerful and necessary tool
- Furthermore, observational studies have tremendous value as initial studies to build up support for larger, more resource-intensive controlled experiments
- However, they can be very misleading — identifying confounders is not always easy, and you can't control for everything

# Racial bias in Florida

- A study of racial bias in the administration of the death penalty was published in the *Florida Law Review*
- The sample consists of 674 defendants convicted of multiple homicides in Florida between 1976 and 1987, classified by the defendant's and the victims' races:

Victims	White defendants		Black defendants	
	Total	Death penalty	Total	Death penalty
White	467	53	48	11
Black	16	0	143	4

## Evidence for racial bias against white defendants

- From the table, the overall percentage of white defendants who received the death penalty is

$$\frac{53 + 0}{467 + 16} = 11.0\%$$

- And for black defendants,

$$\frac{11 + 4}{48 + 143} = 7.9\%$$

- This would seem to be evidence of racial bias against white defendants

## Controlling for victims' race

- However, let's control for the potentially confounding effect of victims' race by calculating the percent who received the death penalty separately for white victims and black victims:

Victims' race	% sentenced to death	
	White	Black
White	11.3	22.9
Black	0.0	2.8

- This table indicates racial bias against black defendants

## What's going on?

- This seems paradoxical: if black defendants are more likely to receive the death penalty for white victims, and also for black victims, how can white defendants be more likely to receive the death penalty overall?
- The answer is that both races are much more likely to be involved in murders in which the victim is the same race as the defendant (97% of white defendants were on trial for the murder of white victims; 75% of black defendants were on trial for the murder of black victims)
- Furthermore, Florida juries were much more likely to award the death penalty in cases involving white victims (12.5%) than black victims (2.5%)
- Thus, the apparent racial bias against white defendants could be due to the confounding factor of the victims' race

## Numerical summaries

- Seeing all the data is clearly valuable, but for the sake of simplicity, it is also useful to summarize a comparison with just a single number
- The most common such summary is the *average*, or *mean*
- The average of a list of numbers equals their sum divided by how many of them there are:

$$\bar{x} = \frac{x_1 + x_2 + \cdots}{n} = \frac{\sum_{i=1}^n x_i}{n}$$

- Thus, the average of 4,5,1, and 9 is:

$$\frac{4 + 5 + 1 + 9}{4} = \frac{19}{4} = 4.75$$

## Percentages are averages

- The percentage is a kind of average, in which we are taking the average of whether something happens (in which case it equals 1) or doesn't happen (in which case it equals 0)
- For example, the percentage of white defendants who received the death penalty is

$$\begin{aligned}\frac{1 + 1 + 0 + 1 + 0 + \dots}{n} &= \frac{\# \text{ received the death penalty}}{\text{total } \# \text{ of white defendants}} \\ &= \frac{53}{483} \\ &= 11.0\%\end{aligned}$$

## Weighted averages

- Another way to think about this overall percentage is that it comes from two sources: trials involving white defendants and trials involving black defendants
- The fraction of white defendants sentenced to the death penalty was 11.3% and 0.0% in these trials, but the overall average isn't just the simple average of these two numbers
- Rather, it is *weighted* by the fraction of trials involved:

$$0.967 \times 11.3\% + 0.033 \times 0.0\% = 11.0\%;$$

note that 0.967 is the fraction of trials with white defendants that involved white victims

## Weighted averages (continued)

- This is known as a *weighted average*, and the concept will appear several times in this course
- In a regular average, every observation gets an equal weight of  $1/n$  — an equivalent way of writing the average is

$$\bar{x} = \sum_{i=1}^n \frac{1}{n} x_i$$

- In a weighted average, observation  $i$  gets weight  $w_i$ :

$$\bar{x} = \sum_{i=1}^n w_i x_i$$

where the weights must add up to 1

# Direct standardization

- Breaking down the average in this way helps to see how we might control for confounding factors
- In reality, white defendants were usually on trial for murdering white victims, and black defendants for murdering black victims... but what if they weren't?
- With a weighted average, we can force these fractions to be the same for each group
- This is known as *direct standardization*

## Direct standardization (continued)

- Direct standardization (also called confounder-balanced comparison) is a way to estimate what the difference between the groups would look like if they had the same distribution of the confounder
- This is the same thing that randomization tries to achieve: if we were to (somehow) randomly assign the defendant a race, then both white and black defendants would be on trial for the murder of a white victim 76.4% of the time (since  $515/674$  trials involved white victims)
- Here, since we can't actually randomize the defendant's race, we have to settle for a mathematical approximation

## Balanced comparison (controlled for victims' race)

- The *unadjusted* death penalty rates were:

$$\text{White: } 0.967 \times 11.3\% + 0.033 \times 0.0\% = 11.0\%$$

$$\text{Black: } 0.251 \times 22.9\% + 0.749 \times 2.8\% = 7.9\%$$

- If we balance the confounder using direct standardization, however, we get

$$\text{White: } 0.764 \times 11.3\% + 0.236 \times 0.0\% = 8.6\%$$

$$\text{Black: } 0.764 \times 22.9\% + 0.236 \times 2.8\% = 18.2\%$$

## Procedure for direct standardization

- To summarize these ideas into a step by step procedure for direct standardization:
  - (1) Identify the *outcome*, the *group* that you are interested in, and the *confounder* you are adjusting for
  - (2) Calculate  $w_1, w_2, \dots, w_n$ , the overall proportion of observations that belong to each level of the confounder  
(NOTE: at this point, the outcome and group have not entered into the calculation in any way)
  - (3) Calculate  $\bar{x}_1, \bar{x}_2, \dots, \bar{x}_n$ , the average (or percentage) for that group at each level of the confounder
  - (4) Calculate the weighted average:  $\bar{x} = \sum_i w_i \bar{x}_i$
- To calculate the standardized rates for additional groups, repeat steps 3 and 4 for that group — steps 1 and 2 remain the same

## Example: Death penalty by victim race

- Earlier we saw that Florida juries awarded the death penalty in 12% of cases involving white victims and 3% of cases involving black victims
- However, this also could be skewed by confounding (here, the race of the defendant)
- **Exercise:** Use direct standardization to obtain death penalty rates for trials involving white victims and for black victims, controlling for the effect of defendant's race

## Example: Death penalty by victim race (cont'd)

- First, we calculate the overall percent of white defendants ( $w_1$ ) and black defendants ( $w_2$ ):

$$w_1 = \frac{16 + 467}{674} = 0.7166$$

$$w_2 = \frac{48 + 143}{674} = 0.2834$$

- Then we can calculate weighted averages for each victim race:

White:  $\bar{x}_1 = 0.7166 \left( \frac{53}{467} \right) + 0.2834 \left( \frac{11}{48} \right) = 15\%$

Black:  $\bar{x}_2 = 0.7166 \left( \frac{0}{16} \right) + 0.2834 \left( \frac{4}{143} \right) = 1\%$

- This calculation indicates a rather extreme bias in the administration of the death penalty in Florida juries of 1976–1987 with respect to the victims' race

# Summary

- Randomized controlled trials are not always possible or practical; for these reasons observational studies also play an important role in science
- Observational studies are always limited by confounding, although known confounders can be accounted for, either through design or statistical calculations
- We have focused on direct standardization; additional approaches to adjusting for confounders are discussed in Regression & ANOVA in Health Sciences (BIOS:5120)