## Randomized experiments

#### INFO/STSCI/ILRST 3900: Causal Inference

3 Sep 2024

At the end of class, you will be able to:

- 1. Explain why exchangeability holds in randomized experiments
- 2. Understand why exchangeability allows for direct estimation of causal effects

### Logistics

#### ▶ Problem Set 1 will be released today

### Potential outcome notation review

- ▶ We typically use *i* to denote a generic unit in our study
- ► Y<sub>i</sub> is the observed outcome for unit i
- $A_i$  is treatment received by unit *i*

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  - The Consistency assumption mean that the outcome we observe corresponds to the potential outcome of the observed treatment
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- ► The causal effect for individual *i* can be defined as

$$Y_i^{\text{treatment}} - Y_i^{\text{NoTreatment}}$$

#### Potential outcome exercise

We observe that Martha ate a Mediterranean diet, and we observe that Martha survived. Suppose Martha had eaten a standard diet, we would have observed that Martha survived.

We observe that Ezra ate a standard diet, and we observe that Ezra did not survive Suppose Ezra had eaten a Mediterranean diet, we would have observed that Ezra survived.

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We observe that Ezra ate a standard diet, and we observe that Ezra did not survive Suppose Ezra had eaten a Mediterranean diet, we would have observed that Ezra survived.

Assuming consistency, what is  $A_i$ ,  $Y_i$ ,  $Y_i^{\text{MedDiet}}$  and  $Y_i^{\text{StanDiet}}$ ,

- When i = Martha?
- When i = Ezra?

#### Potential outcome exercise

Suppose we know the following pieces of information:

- We observe that Martha ate a Mediterranean diet, and we observe that Martha survived. Suppose Martha had eaten a standard diet, we would have observed that Martha survived.
- ► We observe that Ezra ate a standard diet, and we observe that Ezra did not survive

Suppose Ezra had eaten a Mediterranean diet, we would have observed that Ezra survived.

	A <sub>i</sub>	Y <sub>i</sub>	$Y_i^{MedDiet}$	$Y_i^{StanDiet}$
Martha				
Ezra				

# Randomized experiments



<sup>&</sup>lt;sup>1</sup>https://xkcd.com/552/

#### Randomized experiments

The New York Times

TheUpshot

#### What Coronavirus Researchers Can Learn From Economists

Randomized controlled trials remain the gold standard, but natural experiments can help doctors who need answers now.



By Anupam B. Jena and Christopher M. Worsham June 30, 2020



# Fundamental problem of causal inference

- Randomized experiments are the gold standard for estimating causal effects
- Fundamental problem of causal inference is that we don't observe counterfactual outcomes
- Data is still missing in random experiments

	A	<b>Y</b> <sup>a=1</sup>	$  Y^{a=0}$	$  Y^{a=1} - Y^{a=0}$
Ind 1	0	?	0	?
Ind 2	0	?	1	?
Ind 3	0	?	0	?
Ind 4	1	1	?	?
Ind 5	1	0	?	?
Ind 6	1	1	?	?

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► Why do randomized experiments "work"?

Suppose we tested all individuals in Ithaca in the Fall of 2021

- ▶ Whether the individual was vaccinated for Covid A<sub>i</sub> = 1 if vaccinated, A<sub>i</sub> = 0 if not vaccinated
- Whether the individual tested positive for Covid in 2021
  Y<sub>i</sub> = 1 if positive test, Y<sub>i</sub> = 0 if no positive test

# What can go wrong?

#### Front of class

▶ Of the vaccinated individuals (A<sub>i</sub> = 1), 50% had a positive Covid test (Y<sub>i</sub> = 1)

$$\mathsf{E}(Y \mid A = 1) = .5$$

► Of the not vaccinated individuals (A<sub>i</sub> = 0), 70% had a positive Covid test (Y<sub>i</sub> = 1)

$$\mathsf{E}(Y \mid A = 0) = .7$$

How might a vaccine skeptic explain the data?

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#### Back of class

▶ Of the individuals who are vaccinated (A<sub>i</sub> = 1), 70% had a positive Covid test (Y<sub>i</sub> = 1)

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▶ Of the individuals who are **not vaccinated** (A<sub>i</sub> = 0), 50% had a positive Covid test (Y<sub>i</sub> = 1)

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How might a vaccine advocate explain the data?

# Randomized experiment

Experiment designed by Pfizer **randomly assign** each individual (43,000 total) into two groups<sup>2</sup>:

- ► Two doses of BNT162b2 vaccine 21 days apart
- ► Two doses of placebo 21 days apart
- Whether a positive Covid test was recorded between 7 days and 14 weeks after the injection

<sup>&</sup>lt;sup>2</sup>Polack et. al. NEJM 2020

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- ▶ Of the individuals who were given the vaccine (A<sub>i</sub> = 1), 0.04% had a positive Covid test (Y<sub>i</sub> = 1)
- ▶ Of the individuals who were given the placebo (A<sub>i</sub> = 0), 0.9% had a positive Covid test (Y<sub>i</sub> = 1)
- ► Individuals who received the placebo were ≈ 20 times more likely to get Covid

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#### Do the skeptics objections still hold?

<sup>2</sup>Polack et. al. NEJM 2020

#### Why experiments "work"

Table 1. Demographic Characteristics of the Participants in the Main Safety Population.*					
Characteristic	BNT162b2 (N=18,860)	Placebo (N=18,846)	Total (N=37,706)		
Sex — no. (%)					
Male	9,639 (51.1)	9,436 (50.1)	19,075 (50.6)		
Female	9,221 (48.9)	9,410 (49.9)	18,631 (49.4)		
Race or ethnic group — no. (%)†					
White	15,636 (82.9)	15,630 (82.9)	31,266 (82.9)		
Black or African American	1,729 (9.2)	1,763 (9.4)	3,492 (9.3)		
Asian	801 (4.2)	807 (4.3)	1,608 (4.3)		
Native American or Alaska Native	102 (0.5)	99 (0.5)	201 (0.5)		
Native Hawaiian or other Pacific Islander	50 (0.3)	26 (0.1)	76 (0.2)		
Multiracial	449 (2.4)	406 (2.2)	855 (2.3)		
Not reported	93 (0.5)	115 (0.6)	208 (0.6)		
Hispanic or Latinx	5,266 (27.9)	5,277 (28.0)	10,543 (28.0)		
Country — no. (%)					
Argentina	2,883 (15.3)	2,881 (15.3)	5,764 (15.3)		
Brazil	1,145 (6.1)	1,139 (6.0)	2,284 (6.1)		
South Africa	372 (2.0)	372 (2.0)	744 (2.0)		
United States	14,460 (76.7)	14,454 (76.7)	28,914 (76.7)		
Age group — no. (%)					
16-55 yr	10,889 (57.7)	10,896 (57.8)	21,785 (57.8)		
>55 yr	7,971 (42.3)	7,950 (42.2)	15,921 (42.2)		
Age at vaccination — yr					
Median	52.0	52.0	52.0		
Range	16-89	16-91	16-91		
Body-mass index:					
≥30.0: obese	6,556 (34.8)	6,662 (35.3)	13,218 (35.1)		

\* Percentages may not total 100 because of rounding.

† Race or ethnic group was reported by the participants.

The body-mass index is the weight in kilograms divided by the square of the height in meters.

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This is a concept called **exchangeability** 

Sometimes also referred to as exogenous



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- What we would have observed if an individual was given the treatment (Y<sup>a=1</sup>) is independent of whether or not the individual actually received treatment
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- ► What we would have observed if an individual was not given the treatment (Y<sup>a=0</sup>) is independent of whether or not the individual actually received treatment
- Exchangeability means that the **potential** outcomes Y<sup>a</sup> are independent of the observed treatment
- Exchangeability does not mean that the observed outcome Y is independent of the observed treatment!

A = 1 means vaccinated; A = 0 means unvaccinated Y = 1 means covid; Y = 0 means no covid;

	Y <sup>a=1</sup>	Y <sup>a=0</sup>	A	Y
Low Risk 1	0	0	?	?
Low Risk 2	0	0	?	?
High Risk 3	0	1	?	?
High Risk 4	0	1	?	?

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Average Causal Effect = 
$$\underbrace{\mathsf{E}(Y^{a=1})}_{0} - \underbrace{\mathsf{E}(Y^{a=0})}_{1/2} = -1/2$$

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	$Y^{a=1}$	Y <sup>a=0</sup>	A	Y
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Suppose Low Risk choose  $A_i = 0$  and High Risk choose  $A_i = 1$  so the potential outcomes are not independent of the observed treatment

$$\underbrace{\mathsf{E}(Y \mid A = 1)}_{\text{observed vax}} - \underbrace{\mathsf{E}(Y \mid A = 0)}_{\text{observed unvax}} = 0$$

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$$\underbrace{\mathsf{E}(Y \mid A = 1)}_{\text{observed vax}} - \underbrace{\mathsf{E}(Y \mid A = 0)}_{\text{observed unvax}} = -1/2$$

In mathematical notation, exchangeability means

 $\underbrace{Y^{a=1},Y^{a=0}}_{\text{potential outcomes}} \perp \underbrace{\mathcal{A}}_{\text{observed treatment}}$ 

The average causal effect (ACE) is the difference in average outcome that would occur if everyone is treated compared to the average outcome that would occur if no-one is treated

$$ACE = E(Y^{a=1} - Y^{a=0}) = \underbrace{E(Y^{a=1})}_{\text{if everyone is treated}} - \underbrace{E(Y^{a=0})}_{\text{if no-one is treated}}$$

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$$ACE = E(Y^{a=1} - Y^{a=0}) = \underbrace{E(Y^{a=1})}_{\text{if everyone is treated}} - \underbrace{E(Y^{a=0})}_{\text{if no-one is treated}}$$

The problem is, we only know

- ► E(Y<sup>a=1</sup> | A = 1), the average Y<sup>a=1</sup> among individuals who are actually treated
- ► E(Y<sup>a=0</sup> | A = 0), the average Y<sup>a=0</sup> among individuals who are actually not treated

When exchangeability is true, it implies

$$\underbrace{\mathsf{E}(Y^{a=1} \mid A=1)}_{\text{Within treated}} = \underbrace{\mathsf{E}(Y^{a=1} \mid A=0)}_{\text{Within not treated}} = \underbrace{\mathsf{E}(Y^{a=1})}_{\text{everyone}}$$

When exchangeability is true, it implies



This allows us to identify the average causal effect (ACE)



At the end of class, you will be able to:

- 1. Explain why exchangeability holds in randomized experiments
- 2. Understand why exchangeability allows for direct estimation of causal effects