

# **STAT 5000**

**STATISTICAL METHODS I**

**WEEK 3**

**FALL 2024**

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## Unit 1

# MODEL-BASED INFERENCE: HYPOTHESIS TESTING

# HYPOTHESIS TESTING

## Scenario:

- Randomized Experiment
  - ▶ Recruit sample from a single population
  - ▶ Two treatments
  - ▶ Is there a difference in the mean value of the response variable between the two treatments?
- Observational Study
  - ▶ Two populations
  - ▶ One random sample from each population
  - ▶ Is there a difference in the mean value of the variable between the two populations?

# HYPOTHESIS TESTING

## Statistical Assumptions:

- $Y_{11}, Y_{12}, \dots, Y_{1n_1}$  are i.i.d.  $N(\mu_1, \sigma_1^2)$
- $Y_{21}, Y_{22}, \dots, Y_{2n_2}$  are i.i.d.  $N(\mu_2, \sigma_2^2)$
- $Y_{1i}$  and  $Y_{2j}$  are independent for all  $i$  and  $j$
- $\sigma_1^2 = \sigma_2^2 = \sigma^2$

# HYPOTHESIS TESTING

## Key Components:

- Research Question & Hypotheses
- Test Statistic
- Sampling Distribution
- $p$ -value
- Interpretation
- Post-hoc Assessment

# HYPOTHESIS TESTING

## **Research Question & Hypotheses:**

**Research Question:** should be clearly formulated  
i.e., Is this person guilty of committing a crime?

**Null Hypothesis:** denoted  $H_0$   
this is the *status quo*  
i.e., innocent

**Alternative Hypothesis:** denoted  $H_a$   
what you're trying to *prove*  
should be formulated directly from the question  
i.e., guilty

# HYPOTHESIS TESTING

## Hypotheses

- $H_0 : \mu_1 = \mu_2 \quad (\mu_1 - \mu_2 = 0)$
- $H_a :$ 
  - ▶ Left-tailed:  $\mu_1 < \mu_2 \quad (\mu_1 - \mu_2 < 0)$
  - ▶ Right-tailed:  $\mu_1 > \mu_2 \quad (\mu_1 - \mu_2 > 0)$
  - ▶ Two-tailed:  $\mu_1 \neq \mu_2 \quad (\mu_1 - \mu_2 \neq 0)$
  - ▶ Should pick one based on the research question

# HYPOTHESIS TESTING

## Test Statistic

- This is like the *evidence* in the criminal trial example ...
- Compute the *observed value* from the study data

$$t = \frac{\bar{y}_1 - \bar{y}_2}{s_p \sqrt{\frac{1}{n_1} + \frac{1}{n_2}}}$$

- Is it typical to see this value when  $H_0$  is true?
- Or is this value really unlikely when  $H_0$  is true?
- Compare this to a distribution of randomized values

## Sampling Distribution

- Create the distribution of randomized values (assuming  $H_0$  true)

$$T = \frac{\bar{Y}_1 - \bar{Y}_2}{S_p \sqrt{\frac{1}{n_1} + \frac{1}{n_2}}} \sim t_{n_1+n_2-2}$$

- ▶ If  $H_0$  is true,  $\mu_1 - \mu_2 = 0$
- ▶ If  $H_0$  is true, expect to observe  $T$  close to zero.
- ▶ Unlikely to observe large deviations from zero if  $H_0$  true.

# HYPOTHESIS TESTING

## *p*-value

**Definition:** The probability of getting a new test statistic that is at least as extreme as the one observed assuming  $H_0$  is true

- The *p*-value is NOT the probability that  $H_0$  is true
- Use the sampling distribution to compute
- Choose the “extreme” (or tail) based on  $H_a$
- Compute area in tail where  $t$  is the cutoff

# HYPOTHESIS TESTING

## Computing $p$ -values

$$H_a : \mu_1 \neq \mu_2$$

$$p\text{-value} = 2 * P(T_{n_1+n_2-2} > |t|)$$

$$H_a : \mu_1 < \mu_2$$

$$p\text{-value} = P(T_{n_1+n_2-2} < t)$$

$$H_a : \mu_1 > \mu_2$$

$$p\text{-value} = P(T_{n_1+n_2-2} > t)$$

# HYPOTHESIS TESTING

## Interpretation: Scale-of-Evidence Framework

| $p$ -value range    | scale of evidence statement           |
|---------------------|---------------------------------------|
| $p > 0.1$           | little to no evidence for $H_a$       |
| $0.05 < p < 0.1$    | borderline/weak evidence for $H_a$    |
| $0.025 < p < 0.05$  | moderate evidence for $H_a$           |
| $0.001 < p < 0.025$ | substantial/strong evidence for $H_a$ |
| $p < 0.001$         | overwhelming evidence for $H_a$       |

# HYPOTHESIS TESTING

## Interpretation: Statistical-Significance Framework

- Choose your significance level,  $\alpha$
- Reject  $H_0$  if  $p\text{-value} < \alpha$   
There is statistically significant evidence for  $H_a$
- Fail to reject  $H_0$  if  $p\text{-value} \geq \alpha$   
There is no statistically significant evidence for  $H_a$
- Should always state your interpretation in the context of your study

## Post-hoc Assessment: Errors

- If the  $p$ -value was small:
  - ▶  $H_0$  is true and we unluckily/randomly made an error
  - ▶ Type 1 error probability:  $P(\text{reject } H_0 | H_0 \text{ true}) \leq \alpha$
  - ▶  $H_0$  is false (no error committed)
- If the  $p$ -value was large:
  - ▶  $H_a$  is true and we unluckily/randomly made an error
  - ▶ Type 2 error probability:  $P(\text{fail to reject } H_0 | H_0 \text{ false}) = \beta$
  - ▶ The power of a test is  $1 - \beta$
  - ▶  $H_0$  is true (no error committed)

# HYPOTHESIS TESTING

## Example: Lizard Infection Study

- Independent random samples of 15 lizards infected with a disease and 15 non-infected lizards
- Test null hypothesis that mean distance traveled in two minutes is the same for both populations

- t-test of  $H_0 : \mu_1 = \mu_2$  versus  $H_a : \mu_1 \neq \mu_2$

$$t = \frac{-5.3733}{(7.4649)(\sqrt{\frac{1}{15} + \frac{1}{15}})} = -1.97 \text{ on 28 d.f.} \Rightarrow p\text{-value} = 0.0586$$

- t-test of  $H_0 : \mu_1 = \mu_2$  versus  $H_a : \mu_1 < \mu_2$

$$t = \frac{-5.3733}{(7.4649)(\sqrt{\frac{1}{15} + \frac{1}{15}})} = -1.97 \text{ on 28 d.f.} \Rightarrow p\text{-value} = 0.0293$$

# HYPOTHESIS TESTING

## Example: Lizard Infection Study

*T-test for Mean Distance for Two Minute Runs  
Sceloporus Occidentalis Lizards*

*The TTEST Procedure*

*Variable: distance*

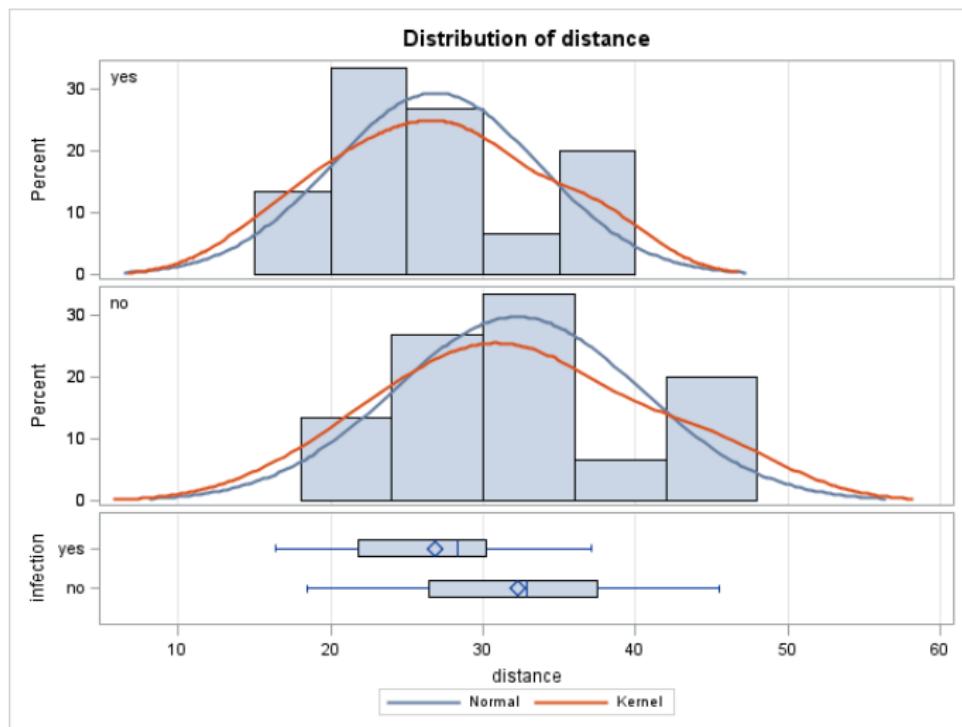
| infection  | N  | Mean    | Std Dev | Std Err | Minimum | Maximum |
|------------|----|---------|---------|---------|---------|---------|
| yes        | 15 | 26.8600 | 6.8096  | 1.7582  | 16.4000 | 37.1000 |
| no         | 15 | 32.2333 | 8.0672  | 2.0829  | 18.4000 | 45.5000 |
| Diff (1-2) |    | -5.3733 | 7.4649  | 2.7258  |         |         |

| infection  | Method        | Mean    | 95% CL Mean | Std Dev | 95% CL Std Dev |
|------------|---------------|---------|-------------|---------|----------------|
| yes        |               | 26.8600 | 23.0889     | 30.6311 | 6.8096         |
| no         |               | 32.2333 | 27.7659     | 36.7008 | 8.0672         |
| Diff (1-2) | Pooled        | -5.3733 | -10.9569    | 0.2102  | 7.4649         |
| Diff (1-2) | Satterthwaite | -5.3733 | -10.9640    | 0.2173  | 5.9240         |
|            |               |         |             |         | 10.0960        |

| Method        | Variances | DF     | t Value | Pr >  t |
|---------------|-----------|--------|---------|---------|
| Pooled        | Equal     | 28     | -1.97   | 0.0586  |
| Satterthwaite | Unequal   | 27.233 | -1.97   | 0.0589  |

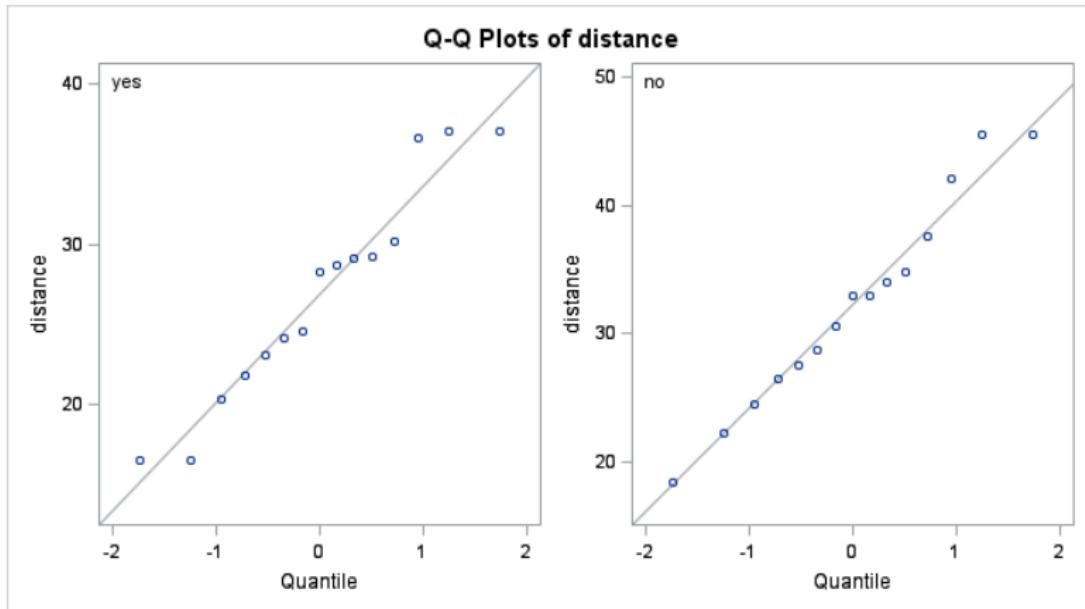
# HYPOTHESIS TESTING

## Example: Lizard Infection Study



# HYPOTHESIS TESTING

## Example: Lizard Infection Study



## Unit 1

# MODEL-BASED INFERENCE: CONFIDENCE INTERVALS

# CONFIDENCE INTERVALS

## Scenario

- Randomized Experiment
  - ▶ Two treatments
  - ▶ Is there a difference in the mean value of the response variable between the two treatments?
- Observational Study
  - ▶ Two populations
  - ▶ One sample from each population
  - ▶ Is there a difference in the mean value of the variable between the two populations?

# CONFIDENCE INTERVALS

## Confidence interval for $\mu_1 - \mu_2$

- Researchers may be interested in estimating the mean difference between the two treatments/populations under study.
- Assumptions
  - ▶  $Y_{11}, Y_{12}, \dots, Y_{1n_1}$  are i.i.d.  $N(\mu_1, \sigma^2)$
  - ▶  $Y_{21}, Y_{22}, \dots, Y_{2n_2}$  are i.i.d.  $N(\mu_2, \sigma^2)$
  - ▶ So, population variances are equal
  - ▶  $Y_{1i}$  and  $Y_{2j}$  are independent for all  $i$  and  $j$

# CONFIDENCE INTERVALS

## Confidence interval for $\mu_1 - \mu_2$

- Based on the assumptions, we have the key result

$$\frac{(\bar{Y}_1 - \bar{Y}_2) - (\mu_1 - \mu_2)}{S_p \sqrt{\frac{1}{n_1} + \frac{1}{n_2}}} \sim t_{n_1+n_2-2}$$

- We can construct a confidence interval using this result ...

# CONFIDENCE INTERVALS

## Confidence interval for $\mu_1 - \mu_2$

- The idea is that, a  $100(1 - \alpha)\%$  confidence interval for  $(\mu_1 - \mu_2)$  contains the values of  $\mu_1 - \mu_2$  for which

$$\left| \frac{(\bar{Y}_1 - \bar{Y}_2) - (\mu_1 - \mu_2)}{S_p \sqrt{\frac{1}{n_1} + \frac{1}{n_2}}} \right| \leq t_{(n_1+n_2-2), 1-\alpha/2}$$

- Solving for  $\mu_1 - \mu_2$  gives

$$(\bar{Y}_1 - \bar{Y}_2) \pm t_{n_1+n_2-2, 1-\alpha/2} S_p \sqrt{\frac{1}{n_1} + \frac{1}{n_2}}$$

# CONFIDENCE INTERVALS

$$\begin{aligned}1 - \alpha &= Pr \left( t_{n_1+n_2-2,\alpha/2} \leq \frac{(\bar{Y}_1 - \bar{Y}_2) - (\mu_1 - \mu_2)}{S_p \sqrt{\frac{1}{n_1} + \frac{1}{n_2}}} \leq t_{n_1+n_2-2,1-\alpha/2} \right) \\&= Pr \left( t_{n_1+n_2-2,\alpha/2} S_p \sqrt{\frac{1}{n_1} + \frac{1}{n_2}} \leq (\bar{Y}_1 - \bar{Y}_2) - (\mu_1 - \mu_2) \leq t_{n_1+n_2-2,1-\alpha/2} S_p \sqrt{\frac{1}{n_1} + \frac{1}{n_2}} \right) \\&= Pr \left( -(\bar{Y}_1 - \bar{Y}_2) + t_{n_1+n_2-2,\alpha/2} S_p \sqrt{\frac{1}{n_1} + \frac{1}{n_2}} \leq -(\mu_1 - \mu_2) \leq -(\bar{Y}_1 - \bar{Y}_2) + t_{n_1+n_2-2,1-\alpha/2} S_p \sqrt{\frac{1}{n_1} + \frac{1}{n_2}} \right)\end{aligned}$$

# CONFIDENCE INTERVALS

$$\begin{aligned}1 - \alpha &= Pr \left( (\bar{Y}_1 - \bar{Y}_2) - t_{n_1+n_2-2, \alpha/2} S_p \sqrt{\frac{1}{n_1} + \frac{1}{n_2}} \geq (\mu_1 - \mu_2) \geq \right. \\&\quad \left. (\bar{Y}_1 - \bar{Y}_2) - t_{n_1+n_2-2, 1-\alpha/2} S_p \sqrt{\frac{1}{n_1} + \frac{1}{n_2}} \right) \\&= Pr \left( (\bar{Y}_1 - \bar{Y}_2) - t_{n_1+n_2-2, 1-\alpha/2} S_p \sqrt{\frac{1}{n_1} + \frac{1}{n_2}} \leq (\mu_1 - \mu_2) \leq \right. \\&\quad \left. (\bar{Y}_1 - \bar{Y}_2) + t_{n_1+n_2-2, 1-\alpha/2} S_p \sqrt{\frac{1}{n_1} + \frac{1}{n_2}} \right)\end{aligned}$$

# CONFIDENCE INTERVALS

## Difference in Means

- Assumptions:
  - ▶  $Y_{11}, Y_{12}, \dots, Y_{1n_1}$  are i.i.d.  $N(\mu_1, \sigma^2)$
  - ▶  $Y_{21}, Y_{22}, \dots, Y_{2n_2}$  are i.i.d.  $N(\mu_2, \sigma^2)$
  - ▶ So, population variances are equal
  - ▶  $Y_{1i}$  and  $Y_{2j}$  are independent for all  $i$  and  $j$
- Then, a  $100(1 - \alpha)\%$  confidence interval for  $\mu_1 - \mu_2$  is

$$(\bar{Y}_1 - \bar{Y}_2) \pm t_{n_1+n_2-2, 1-\alpha/2} S_p \sqrt{\frac{1}{n_1} + \frac{1}{n_2}}$$

$$\text{where } S_p = \frac{(n_1 - 1)S_1^2 + (n_2 - 1)S_2^2}{n_1 + n_2 - 2}$$

# CONFIDENCE INTERVALS

## Relationship Between Tests and Confidence Intervals

- Let  $\delta = \mu_1 - \mu_2$  (A.K.A. the true effect size)
- A two-sided  $t$ -test will reject  $H_0 : \delta = \mu_1 - \mu_2 = 0$  at the  $\alpha$  level if and only if  $0$  is not in  $100(1 - \alpha)\%$  confidence interval for  $\delta = \mu_1 - \mu_2$ .
- A  $100(1 - \alpha)\%$  confidence interval can be constructed by including all values of  $\delta$  such that data does not provide sufficient evidence to reject the null hypothesis  
 $H_0 : \mu_1 - \mu_2 = \delta$  relative to the two-sided alternative  
 $H_a : \mu_1 - \mu_2 \neq \delta$  at the  $\alpha$  significance level.

# CONFIDENCE INTERVALS

## Frequentist Interpretation

- $\mu_1, \mu_2, \sigma$  are fixed unknowns
- $\bar{Y}_1, \bar{Y}_2, S_p$  are random variables
- A confidence interval is a *random* quantity
- Across a large number of repeated samples,  $100(1 - \alpha)\%$  of such intervals will contain the true value of  $\mu_1 - \mu_2$  (a long-term frequency property).
- Any particular interval either contains the true value of  $\mu_1 - \mu_2$  or not.
- The  $100(1 - \alpha)\%$  probability describes the process of constructing the intervals.

# CONFIDENCE INTERVALS

## More Details ...

- Confidence interval widths depend on
  - ▶ the confidence level (which is related to significance  $\alpha$ )
  - ▶ the value of  $\sigma$
  - ▶ sample sizes  $n_1$  and  $n_2$
- For the lizard study, a 95% confidence interval for  $\mu_1 - \mu_2$  is
$$-5.3733 \pm (2.048)(7.4649) \sqrt{\frac{1}{15} + \frac{1}{15}} \Rightarrow (-10.96, 0.21)$$

# CONFIDENCE INTERVALS

## *T-test for Mean Distance for Two Minute Runs Sceloporus Occidentalis Lizards*

### *The TTTEST Procedure*

*Variable: distance*

| infection  | N  | Mean    | Std Dev | Std Err | Minimum | Maximum |
|------------|----|---------|---------|---------|---------|---------|
| yes        | 15 | 26.8600 | 6.8096  | 1.7582  | 16.4000 | 37.1000 |
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| infection  | Method        | Mean    | 95% CL Mean | Std Dev | 95% CL Std Dev        |
|------------|---------------|---------|-------------|---------|-----------------------|
| yes        |               | 26.8600 | 23.0889     | 30.6311 | 6.8096 4.9855 10.7395 |
| no         |               | 32.2333 | 27.7659     | 36.7008 | 8.0672 5.9062 12.7228 |
| Diff (1-2) | Pooled        | -5.3733 | -10.9569    | 0.2102  | 7.4649 5.9240 10.0960 |
| Diff (1-2) | Satterthwaite | -5.3733 | -10.9640    | 0.2173  |                       |

| Method        | Variances | DF     | t Value | Pr >  t |
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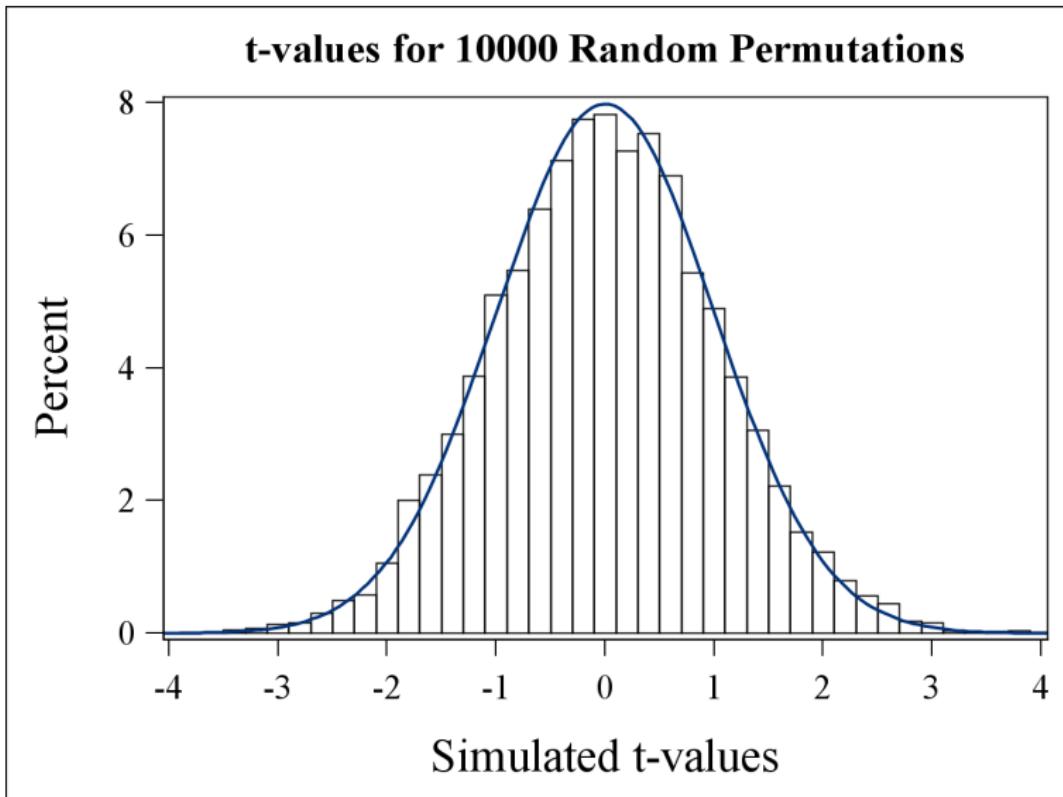
| Equality of Variances |        |        |         |        |
|-----------------------|--------|--------|---------|--------|
| Method                | Num DF | Den DF | F Value | Pr > F |
| Folded F              | 14     | 14     | 1.40    | 0.5343 |

# CONFIDENCE INTERVALS

## Comparison of Randomization and Model Based Approaches

- Randomization tests
  - ▶ Require no model for population distributions
  - ▶ Are appropriate for randomized experiments
  - ▶ Require more computation
- Randomization CI's (based on test/interval relationship)  
require even more computation
- Model-based tests and CIs provide easily evaluated approximations to randomization inference
- Model-based approach can be useful for study design

# CONFIDENCE INTERVALS



## Unit 1

# MODEL-BASED INFERENCE: SAMPLE SIZE CONSIDERATIONS

## Sample Size Determination

- Frequently asked design questions:  
How many experimental units should I use?
- Four possible considerations:
  1. Include as many as you can afford or find
  2. Desired precision (standard error) of some estimator
  3. Width of a confidence interval
  4. Power of a hypothesis test

# SAMPLE SIZE

## Based on Standard Error

### Difference in Means

- Difference in population means ( $\mu_1 - \mu_2$ ):  
 $s.e.(\bar{Y}_1 - \bar{Y}_2) = S_p \sqrt{1/n_1 + 1/n_2}$
- Assuming  $n_1 = n_2 = n$ , we have:  
 $s.e.(\bar{Y}_1 - \bar{Y}_2) = S_p \sqrt{2/n}$
- Specify an acceptable value for the s.e. and solve for  $n$   
 $s.e. = \frac{\sqrt{2}S_p}{\sqrt{n}} \Rightarrow n = \frac{2S_p^2}{(s.e.)^2}$
- Requires a value for  $S_p$  from:
  - ▶ a previous study
  - ▶ a pilot study
  - ▶ a guess

# SAMPLE SIZE

## Based on Confidence Interval

### Difference in Means

- Width of CI (assuming  $n_1 = n_2 = n$ ) is

$$w = 2 \times t_{2(n-1), 1-\alpha/2} S_p \sqrt{2/n}$$

- Find  $n$  to achieve specified width

$$n = 8 \left( \frac{t_{2(n-1), 1-\alpha/2} S_p}{w} \right)^2$$

- One difficulty is that  $n$  enters twice (sample size and df for t)

- Compute initial value using  $z_{1-\alpha/2}$  in place of the t-value

$$n_0 = 8 \left( \frac{z_{1-\alpha/2} S_p}{w} \right)^2$$

- Then improve using  $n = 8 \left( \frac{t_{2(n_0-1), 1-\alpha/2} S_p}{w} \right)^2$

# SAMPLE SIZE

## Based on Hypothesis Test

Recall:

Four Possible Outcomes for Hypothesis Test

| Decision             | $H_0$ is true       | $H_0$ is false       |
|----------------------|---------------------|----------------------|
| Reject $H_0$         | <b>Type I Error</b> | Good Decision        |
| Fail to reject $H_0$ | Good Decision       | <b>Type II Error</b> |

## Recall: Hypothesis Testing Errors

- Type I error  $\Rightarrow$  reject  $H_0$  when it is true
  - ▶ Probability of Type I error:  $\alpha = P(\text{reject } H_0 \mid H_0 \text{ is true})$
  - ▶ Threshold for p-value, significance level
  - ▶ Value of  $\alpha$  is typically set to be small, such as 0.05.
- Type II error  $\Rightarrow$  fail to reject  $H_0$  when it is false
  - ▶ Probability of Type II error:  $\beta = P(\text{fail to reject } H_0 \mid H_0 \text{ is false})$

# SAMPLE SIZE

## Recall: Power

- Power of a statistical test =  $1 - \beta$
- Function of a particular alternative to the null hypothesis:  
 $\text{Power} = 1 - \beta = P(\text{reject } H_0 | H_a \text{ true})$
- For fixed  $\alpha$ , power is determined by
  - ▶ How much the alternative deviates from the null hypothesis  
(true effect size, e.g.,  $\delta = \mu_1 - \mu_2$ )
  - ▶ population variance ( $\sigma^2$ )
  - ▶ sample sizes ( $n_1, n_2$ )

*We can use power to determine the sample size because, for any given test, type I error rate  $\alpha$ , power  $1 - \beta$ , standard deviation  $\sigma$  or  $S$ , effect size  $\delta = \mu_1 - \mu_2$ , and sample size  $n$  are all related. Specifying four enables us to calculate the fifth.*

# SAMPLE SIZE

## Based on Hypothesis Test

### Difference in Means

For a t-test of  $H_0 : \mu_1 = \mu_2$  against  $H_a : \mu_1 \neq \mu_2$

- Equal sample sizes  $n_1 = n_2 = n$ ,
- Type I error rate  $= \alpha$ ,
- Power  $= 1 - \beta$  for detecting  $\delta = \mu_1 - \mu_2$ ,
- Pooled estimate of the population variance  $S_p^2$

the required sample size for each group is

$$n = \frac{(t_{2(n-1), 1-\alpha/2} + t_{2(n-1), 1-\beta})^2 (2S_p^2)}{\delta^2}$$

## Based on Hypothesis Test

### Difference in Means

- As before,  $n$  enters twice. Use the same two-step approach.

First compute  $n_0 = \frac{(z_{1-\alpha/2} + z_{1-\beta})^2 (2S_p^2)}{\delta^2}$

Then update  $n = \frac{(t_{2(n_0-1), 1-\alpha/2} + t_{2(n_0-1), 1-\beta})^2 (2S_p^2)}{\delta^2}$

- Common to use power values of 80%, 90% or 95%. Just as arbitrary as using  $\alpha=5\%$ .
- Can adapt to one-sided alternative by replacing  $\alpha/2$  with  $\alpha$  in the previous formulas

# SAMPLE SIZE

## Derivation:

- Based on the model assumptions, we have the key result

$$\frac{(\bar{Y}_1 - \bar{Y}_2) - (\mu_1 - \mu_2)}{S_p \sqrt{\frac{1}{n_1} + \frac{1}{n_2}}} \sim t_{n_1+n_2-2}$$

- While controlling the type I error rate at  $\alpha$ , we reject  $H_0$  if

$$\left| \frac{(\bar{Y}_1 - \bar{Y}_2) - 0}{\sqrt{S_p^2 \left( \frac{1}{n_1} + \frac{1}{n_2} \right)}} \right| > t_{(n_1+n_2-2), 1-\alpha/2}$$

# SAMPLE SIZE

## Derivation:

$$\begin{aligned}1 - \beta &= P(\text{reject } H_0 : \mu_1 - \mu_2 = 0 \mid \mu_1 - \mu_2 = \delta) \\&= P\left(\left|\frac{(\bar{Y}_1 - \bar{Y}_2) - 0}{\sqrt{S_p^2\left(\frac{1}{n_1} + \frac{1}{n_2}\right)}}\right| > t_{(n_1+n_2-2), 1-\alpha/2} \mid \mu_1 - \mu_2 = \delta\right) \\&= P\left(|(\bar{Y}_1 - \bar{Y}_2) - 0| > t_{(n_1+n_2-2), 1-\alpha/2} \sqrt{S_p^2\left(\frac{1}{n_1} + \frac{1}{n_2}\right)} \mid \mu_1 - \mu_2 = \delta\right) \\&= P\left((\bar{Y}_1 - \bar{Y}_2) > t_{(n_1+n_2-2), 1-\alpha/2} \sqrt{S_p^2\left(\frac{1}{n_1} + \frac{1}{n_2}\right)} \mid \mu_1 - \mu_2 = \delta\right) \\&\quad + P\left((\bar{Y}_1 - \bar{Y}_2) < -t_{(n_1+n_2-2), 1-\alpha/2} \sqrt{S_p^2\left(\frac{1}{n_1} + \frac{1}{n_2}\right)} \mid \mu_1 - \mu_2 = \delta\right)\end{aligned}$$

# SAMPLE SIZE

## Derivation:

$$1 - \beta =$$

$$P \left( \frac{(\bar{Y}_1 - \bar{Y}_2) - \delta}{\sqrt{S_p^2(\frac{1}{n_1} + \frac{1}{n_2})}} > \frac{t_{(n_1+n_2-2), 1-\alpha/2} \sqrt{S_p^2(\frac{1}{n_1} + \frac{1}{n_2})} - \delta}{\sqrt{S_p^2(\frac{1}{n_1} + \frac{1}{n_2})}} \mid \mu_1 - \mu_2 = \delta \right)$$

$$+ P \left( \frac{(\bar{Y}_1 - \bar{Y}_2) - \delta}{\sqrt{S_p^2(\frac{1}{n_1} + \frac{1}{n_2})}} < \frac{-t_{(n_1+n_2-2), 1-\alpha/2} \sqrt{S_p^2(\frac{1}{n_1} + \frac{1}{n_2})} - \delta}{\sqrt{S_p^2(\frac{1}{n_1} + \frac{1}{n_2})}} \mid \mu_1 - \mu_2 = \delta \right)$$

This implies that

$$-t_{(n_1+n_2-2), 1-\beta} \approx \frac{t_{(n_1+n_2-2), 1-\alpha/2} \sqrt{S_p^2(\frac{1}{n_1} + \frac{1}{n_2})} - \delta}{\sqrt{S_p^2(\frac{1}{n_1} + \frac{1}{n_2})}}$$

# SAMPLE SIZE

## Derivation:

- With  $n_1 = n$  and  $n_2 = Cn$  and  $\delta > 0$  we have

$$-t_{((1+C)n-2), 1-\beta} \approx \frac{t_{((1+C)n-2), 1-\alpha/2} \sqrt{S_p^2(\frac{1}{n}(1 + \frac{1}{C}))} - \delta}{\sqrt{S_p^2(\frac{1}{n}(1 + \frac{1}{C}))}}$$

- Solve for  $n$ :

$$n = \frac{(t_{((1+C)n-2), 1-\alpha/2} + t_{((1+C)n-2), 1-\beta})^2 S_p^2(1 + \frac{1}{C})}{\delta^2}$$

- Setting  $n_1 = n_2 = n$ , we get

$$n = \frac{(t_{2(n-1), 1-\alpha/2} + t_{2(n-1), 1-\beta})^2 2S_p^2}{\delta^2}$$

# SAMPLE SIZE

## Example: Bone Density Study

- Client's request:  
*I want to do a randomized experiment to compare two treatments for preventing bone loss in elderly women. I want to use a 0.05-level t-test that has at least an 80% chance of detecting of a difference of 4 units between mean loss in bone density for the two treatments. How many subjects do I need?*
- After some discussion the client explains that she would like to use treatment groups of the same size

# SAMPLE SIZE

## Example: Bone Density Study

- Further discussion about previous studies of bone density loss in elderly women suggests that the measured responses should be approximately normally distributed for each treatment  $\Rightarrow$  a t-test could be used
- The null hypothesis is  $H_0 : \mu_1 = \mu_2$
- The alternative is two-sided and the deviation of interest is  $\delta = |\mu_1 - \mu_2| = 4$
- Type I error level is  $\alpha = 0.05$
- Power =  $1-\beta = 0.80$ , and  $\beta=0.20$
- Review of previous studies suggests  $S_p^2 \approx 25$

# SAMPLE SIZE

## Example: Bone Density Study

- Compute initial sample size value

$$\begin{aligned} n_0 &= \frac{(z_{0.975} + z_{0.80})^2 (2S_p^2)}{\delta^2} \\ &= \frac{(1.96 + 0.841)^2 (2 \times 25)}{(4)^2} \\ &= 24.52 \\ &\Rightarrow 25 \end{aligned}$$

# SAMPLE SIZE

## Example: Bone Density Study

- Then compute

$$\begin{aligned} n &= \frac{(t_{48,0.975} + t_{48,0.80})^2 (2S_p^2)}{\delta^2} \\ &= \frac{(2.01 + 0.849)^2 (2 \times 25)}{(4)^2} \\ &= 25.54 \\ &\Rightarrow 26 \end{aligned}$$

- Use 26 subjects in each treatment group

# SAMPLE SIZE

## Example: Bone Density Study

*Instead of performing a test, suppose the client in the previous example wanted to construct a 95% confidence interval for the difference in the mean bone density loss for the two treatments of width 4 units*

Goal:  $(\bar{Y}_1 - \bar{Y}_2) \pm 2$

- Width of CI (assuming  $n_1 = n_2 = n$ ) is

$$w = 2 \times t_{2(n-1), 1-\alpha/2} S_p \sqrt{2/n}$$

- Find  $n$  to achieve specified width

$$n = 8 \left( \frac{t_{2(n-1), 1-\alpha/2} S_p}{w} \right)^2$$

# SAMPLE SIZE

## Example: Bone Density Study

- $\alpha = 0.05$  and  $S_p^2 \approx 25$

- First compute

$$n_0 = 8 \left( \frac{z_{0.975} \times S_p}{w} \right)^2 = 8 \left( \frac{1.96 \times 5}{4} \right)^2 = 48.02 \Rightarrow 49$$

- Then improve using

$$n = 8 \left( \frac{t_{96,0.975} \times S_p}{w} \right)^2 = 8 \left( \frac{1.99 \times 5}{4} \right)^2 = 49.50 \Rightarrow 50$$

- Use 50 subjects in each group

# SAMPLE SIZE

## Example: Bone Density Study

Suppose the client simply wanted to use enough subjects to make the standard error for the estimated difference of the means no larger than 1.0.

$$\text{Goal: } s.e.(\bar{Y}_1 - \bar{Y}_2) = S_p \sqrt{\frac{2}{n}} = 1$$

- $S_p^2 \approx 25$
- Compute  $n = \frac{2 \times S_p^2}{(1)^2} = \frac{2(25)}{1} = 50$
- Use 50 subjects in each group

## Designing Comparative Studies

- Consider unequal group sizes in experiments when
  - ▶ One treatment is much more expensive than another
  - ▶ One treatment is limited or not readily available
  - ▶ Variation in responses differs between treatments
- Unequal sample sizes in observational studies are more common
- Previously only considered  $n_1 = n_2 = n$ , why?
  - ▶ Could consider  $n_1 = 2n_2$ , same concept, modified formula
  - ▶ For fixed value of  $n_1 + n_2$ ,  $n_1 = n_2$  gives estimator of difference in means with smallest s.e.  
(assuming homogeneous variances)

## Designing Comparative Studies

- Distribution of the t-statistic is not distorted by unequal variances when  $n_1 = n_2$
- Size for one group may be limited by availability or higher cost, but

$n_1 = 10, n_2 = 40$  is better than  $n_1 = 10, n_2 = 10$

$n_1 = 10, n_2 = 40$  is not as good as  $n_1 = 25, n_2 = 25$

## QUESTIONS?

Contact me:

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