

# Statistics 305/605: Introduction to Biostatistical Methods for Health Sciences

## Chapter 14 : Inference for Proportions

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# Inference for Proportions (Chapter 14)

- ▶ Instead of quantitative measurements, we classify each sampled individual into one of two categories.
  - ▶ success, failure (canonical)
  - ▶ breast cancer, no breast cancer
  - ▶ Etc.
- ▶ Want to make inference about the proportion  $p$  of successes in a population, or about the difference between the proportions  $p_1$  and  $p_2$  of successes in two populations.

## Example: Women's Health Initiative (WHI)

- ▶ A randomized controlled trial, called the Women's Health Initiative, randomized 16,608 post-menopausal women aged 50-79 years to receive either hormone replacement therapy in the form of estrogen plus progestin (EP;  $n_1 = 8506$ ), or a placebo ( $n_2 = 8102$ ).
- ▶ After five years, 166 of those in the EP group had developed invasive breast cancer, compared to 122 in the placebo group.
- ▶ The populations to compare are postmenopausal women aged 50-79 years, who are taking EP (population 1) or placebo (population 2).
- ▶ The sample proportions are  $\hat{p}_1 = 166/8506 = 0.0195$  and  $\hat{p}_2 = 122/8102 = 0.0151$
- ▶ It looks like the EP group has a higher risk of breast cancer, but could this difference be due to chance?

# Outline of Approach

- ▶ Similar approach to inference of population means (quantitative outcomes), with some minor differences.
- ▶ Inference is based on the sampling distribution of  $\hat{p}_1 - \hat{p}_2$
- ▶ Two-stage reasoning:
  1. Transform the statistic  $\hat{p}_1 - \hat{p}_2$  into an initial pivotal quantity,  $Z_1$ , whose denominator depends on the unknown parameters  $p_1$  and  $p_2$ .
  2. Get a final pivotal quantity,  $Z$ , by replacing the unknown  $p_i$ 's in the denominator of  $Z_1$  with estimates.
- ▶ CIs and hypothesis tests follow from the approximate sampling distribution of the final pivotal quantity,  $Z$ .
- ▶ Note: Not covering *Inference for a single proportion* (text, sections 14.2 – 14.5).

## Sampling Distribution of $\hat{p}_1 - \hat{p}_2$

- ▶ Assume that we have independent simple random samples (SRSs) of size  $n_1$  and  $n_2$ , from the two parent populations.
- ▶ Then the distribution of  $\hat{p}_1 - \hat{p}_2$  has
  - ▶ mean  $p_1 - p_2$  and
  - ▶ SD

$$\sqrt{p_1(1 - p_1)/n_1 + p_2(1 - p_2)/n_2}.$$

- ▶ If the sample sizes are “large”, the CLT tells us that the shape of this distribution is approximately normal.
  - ▶ (The CLT applies because it turns out that a proportion is an average ... of 0's and 1's.)

# Initial Transformation

- ▶ For large samples,  $\hat{p}_1 - \hat{p}_2$  is **approximately** normally distributed with
  - ▶ mean  $p_1 - p_2$ , and
  - ▶ SD

$$\sqrt{p_1(1 - p_1)/n_1 + p_2(1 - p_2)/n_2}$$

- ▶ So the distribution of

$$Z_1 = \frac{(\hat{p}_1 - \hat{p}_2) - (p_1 - p_2)}{\sqrt{p_1(1 - p_1)/n_1 + p_2(1 - p_2)/n_2}}$$

is **approximately**  $N(0, 1)$ .

## $Z_1$ with Estimated SDs

- ▶ Inserting the estimates  $\hat{p}_1$  and  $\hat{p}_2$  for the unknown parameters  $p_1$  and  $p_2$  into  $Z_1$  above gives

$$Z = \frac{(\hat{p}_1 - \hat{p}_2) - (p_1 - p_2)}{\sqrt{\hat{p}_1(1 - \hat{p}_1)/n_1 + \hat{p}_2(1 - \hat{p}_2)/n_2}}$$

- ▶ What is the distribution of  $Z$ ?
  - ▶ Under certain conditions (see below) it is reasonable to assume that  $Z$  is approximately normal.

# Rule of Thumb for Normal Approximation

- ▶ The normal approximation to the distribution of  $Z$  is considered reliable when the sample sizes  $n_1$  and  $n_2$  are “large”
- ▶ The definition of large depends on the underlying  $p_j$ 's. The text (page 324) suggests:
  - ▶  $n_1 p_1 \geq 5$  and  $n_1(1 - p_1) \geq 5$  and
  - ▶  $n_2 p_2 \geq 5$  and  $n_2(1 - p_2) \geq 5$
- ▶ The parameters  $p_1$  and  $p_2$  are not known so we insert the estimates  $\hat{p}_1 = (\text{number of successes in sample 1})/n_1$  and  $\hat{p}_2 = (\text{number of successes in sample 2})/n_2$ .
- ▶ After inserting estimates, one can simplify the requirements to the following rule:
  - ▶ The normal approximation is reliable when there are at least 5 successes *and* 5 failures in both sample 1 and sample 2.



# Checking rule of thumb for WHI Data

- ▶ At least 5 successes and failures in both samples.
  - ▶ True: 166 cancer, 8340 cancer-free in the EP group; 122 cancer, 7980 cancer-free in the placebo group.

# Confidence Intervals

- ▶ The level- $C$  CI for  $p_1 - p_2$  is of the form:

estimate  $\pm$  margin of error

- ▶ The estimate is  $\hat{p}_1 - \hat{p}_2$
- ▶ The margin of error is  $z^* \times SE$  where
  - ▶  $z^*$  is the upper  $(1 - C)/2$  critical value of the standard normal distribution.
  - ▶  $SE$  is the estimated SD of  $\hat{p}_1 - \hat{p}_2$  in the denominator of  $Z$ ; namely,  $SE = \sqrt{\hat{p}_1(1 - \hat{p}_1)/n_1 + \hat{p}_2(1 - \hat{p}_2)/n_2}$

## Example (WHI)

- ▶ Recall: 16,608 women aged 50-79 years randomized to receive either estrogen plus progestin (EP;  $n_1 = 8506$ ), or a placebo ( $n_2 = 8102$ ). After five years, 166 in the EP group developed invasive breast cancer, compared to 122 in placebo group.
- ▶ For EP,  $\hat{p}_1 = 166/8506$  and, for placebo,  $\hat{p}_2 = 122/8102$ .
- ▶ 95% CI is estimate  $\pm$  margin of error, where
  - ▶ estimate of  $p_1 - p_2$  is  $\hat{p}_1 - \hat{p}_2 = 0.0044$
  - ▶ margin of error is a critical value times standard error of difference.
- ▶ The critical value is 1.96 (see R demo).
- ▶ The standard error is
$$\sqrt{\hat{p}_1(1 - \hat{p}_1)/n_1 + \hat{p}_2(1 - \hat{p}_2)/n_2} = 0.002$$
- ▶ The margin of error is therefore  $1.96 * .002 = 0.00392$ .
- ▶ Putting it all together, the CI is  $0.0044 \pm 0.00392$  or approximately (0.0005, 0.008).

## Test Statistic

- ▶ The null hypothesis is  $H_0 : p_1 - p_2 = 0$ .
- ▶ Numerator of the test statistic is therefore the estimated difference  $(\hat{p}_1 - \hat{p}_2)$  minus 0.
- ▶ Denominator of the test statistic is

$$SE = \sqrt{\hat{p}_1(1 - \hat{p}_1)/n_1 + \hat{p}_2(1 - \hat{p}_2)/n_2}$$

- ▶ BUT, under  $H_0$ , we have  $p_1 = p_2$ . Call this common value  $p$ .
- ▶ Assuming a common proportion  $p$  in the two populations, we pool the 2 samples to obtain an estimate  $\hat{p}$ ; i.e.,

$$\hat{p} = (\text{number of cancers in both samples}) / (n_1 + n_2).$$

- ▶ The formula for the SE of  $\hat{p}_1 - \hat{p}_2$  simplifies to  $\sqrt{\hat{p}(1 - \hat{p}) \times (1/n_1 + 1/n_2)}$
- ▶ So the statistic for testing  $H_0 : p_1 - p_2 = 0$  is

$$Z = \frac{(\hat{p}_1 - \hat{p}_2)}{\sqrt{\hat{p}(1 - \hat{p}) (1/n_1 + 1/n_2)}}$$

## Example (WHL, continued)

- ▶  $H_0 : p_1 - p_2 = 0$ .
- ▶ Test statistic numerator is  $\hat{p}_1 - \hat{p}_2 = 0.0044$ .
- ▶ Test statistic denominator is the SE based on the pooled estimate of  $p = p_1 = p_2$ .
  - ▶ Pooled estimate of the common population proportion is  $\hat{p} = (166 + 122)/(8506 + 8102) = 0.0173$ .
  - ▶ So the SE is

$$\begin{aligned} & \sqrt{\hat{p}(1 - \hat{p})(1/n_1 + 1/n_2)} \\ &= \sqrt{0.0173(1 - 0.0173)(1/8506 + 1/8102)} \\ &= 0.002. \end{aligned}$$

- ▶ The test statistic value is  $z = 0.0044/0.002 = 2.2$ .

## $p$ -value

- ▶ The  $p$ -value is the chance of a value of the test statistic that is as or more extreme than what we did observe in our data, when the null hypothesis is true.
- ▶ Same logic as we saw earlier for inference of population means (Chapter 11).
- ▶ Let  $Z$  be a standard normal random variable and  $z$  be the observed value of the test statistic.
- ▶ For  $H_a : p_1 - p_2 \neq 0$ , the  $p$ -value is  $p = 2P(Z \geq |z|)$ .

## Example (WHI, continued)

- ▶ Suppose we wish to test  
 $H_0: p_1 - p_2 = 0$  vs.  $H_a: p_1 - p_2 \neq 0$  at level  $\alpha = 0.05$ .
- ▶ For an observed value of the test statistic  $z = 2.2$ , computer software calculates a pvalue of about 0.03 (see R demo)
- ▶ We therefore reject  $H_0$  at the 5% level: There is statistical evidence that women taking EP have a higher risk of invasive breast cancer than those taking the placebo.
  - ▶ Note: The EP group appears to have a higher risk of breast cancer than the placebo group because  $\hat{p}_1$  (for EP) is greater than  $\hat{p}_2$  (for placebo), as reflected by  $z = 2.2$  being greater than zero.

# Summary

- ▶ Inference for the difference  $p_1 - p_2$  between two population proportions is based on a pivotal quantity.
- ▶ Confidence intervals are of the form **estimate**  $\pm$  **margin of error**, where
  - ▶ estimate is difference between sample proportions, and
  - ▶ margin of error is a critical value times the standard error of the difference in sample proportions
- ▶ To test the null hypothesis  $H_0 : p_1 - p_2 = 0$  against an alternative  $H_a$  we calculate a test statistic and the  $p$ -value
  - ▶ The  $p$ -value is the chance of seeing a value of the test statistic as or more extreme than the value that was observed in our data, under the null hypothesis.
  - ▶ Compare the  $p$ -value to a significance level  $\alpha$  to obtain a statistical hypothesis test
- ▶ The statistical inference is considered reliable when there are at least 5 successes and 5 failures in each sample.