# Statistical Inference for Two-sample Categorical Data

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#### Outline

- ▶ Video #1
  - ► Two-sample Categorical Data (example and concepts)
- ▶ Video #2
  - ► The Two-sample Z-test for a Difference in Proportions
- ► Video #3
  - Odds Ratios and Confidence Intervals

#### Introduction

- So far in this course all our applications of statistical inference have focused on *one-sample* (one group) settings
  - ▶ We've used Normal models (the Z-test) for inference involving one proportion
  - We've used the t-distribution (the T-test) for inference involving one mean
- This week we will learn how to extend these ideas to two-sample settings, or situations involving the comparison of two proportions or two means

## Surgical Site Infections

- ▶ In the 1860's, surgeries often led to infections that resulted in death
- At the time, experts believed these infections were due to "bad air"
  - Hospitals had policies that required their wards open their windows at midday to air out
- ▶ It was customary for surgeons to move quickly from patient to patient without any sort of special precautions
  - In fact, many took pride the accumulated stains on their surgical gowns as a measure of experience

## Louis Pasteur and Joseph Lister

- In 1862, Louis Pasteur discovered that food spoilage was caused by the growth and proliferation of harmful micro-organisms
- ► Pasteur identified three methods for eliminating these micro-organisms: heat, filtration, and chemical disinfectants
  - ► The method of heating became known as pasteurization (named for Pasteur) and is widely applied to milk, beer, and many other food products

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  - ► The method of heating became known as pasteurization (named for Pasteur) and is widely applied to milk, beer, and many other food products
- Joseph Lister, a Professor of Surgery at the Glasgow Royal Infirmary, became aware of Pasteur's work and theorized that it might explain the infections that frequently occurred after surgery
  - How would you recommend Lister evaluate his theory?



## Lister's Experiment

- Lister proposed a new "sterile" protocol where surgeons were required to wash their hands, wear clean gloves, and disinfect their instruments with a carbolic acid solution
  - ► He randomly assigned 75 patients undergoing surgery to receive either his new "sterile" protocol or be in a control group
  - He then tracked the survival of patients until their discharge from the hospital

	Died	Survived
Control	16	19
Sterile	6	34

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- Bias? Probably not, even though double-blinding wasn't possible, it's unlikely the measurement of the outcome (survival) was biased. It's also unlikely that this is a non-representative group of patients (sampling bias)
- 2. Confounding variables? No, we'd expect random assignment to have balanced the two groups
- 3. Random chance? ... This is where hypothesis testing is useful

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  - In words, what should the null model be for Lister's experiment?
- The null model is that the Lister's proposed sterilization procedure makes no difference
  - That is, equal proportions of the "Sterile" and "Control" groups are expected to die prior to discharge

$$H_0: p_1-p_2=0$$

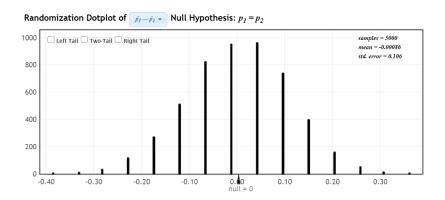
 $\triangleright$  Here,  $p_1$  denotes the proportion of deaths among the "Control" group, and  $p_2$  is the proportion of deaths among the "Sterile" group

## Simulating the Null Distribution

- ▶ If the sterilization protocol made no difference, any deaths observed in this study data occurred at random (ie: the assigned group made no difference)
  - ▶ Thus, under the null model, we can assume the *overall death* rate (estimated by 22/75, or 29%) applies equally to both groups
- We can use StatKey to simulate possible outcomes that could occur under this null model using sets of  $n_1 = 35$  and  $n_2 = 40$ "weighted coin-flips", where each flip represents a 29% chance of death

# Simulating the Null Distribution

If both groups had the same death rate (29%), we could expect to have observed the following differences in proportions:



The study saw a difference of  $\hat{p}_1 - \hat{p}_2 = 16/35 - 6/40 = 0.31$ , only 2 of 5000 simulated outcomes were this extreme!



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  - Central Limit theorem (combined with some probability theory) and linear algebra) suggests the following:

$$\hat{p}_1 - \hat{p}_2 \sim N\left(p_1 - p_2, \sqrt{\frac{p_1(1-p_1)}{n_1} + \frac{p_2(1-p_2)}{n_2}}\right)$$

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- One challenge in applying this theoretical result is that our null hypothesis only specifies that  $p_1 = p_2$  (which can be satisfied by many different values)
  - The most common solution is to used the pooled (overall) proportion in place of both  $p_1$  and  $p_2$
  - In our example, this would be applying the overall death rate of 29% to both groups



- 1. State the null hypothesis (ie:  $H_0$ :  $p_1 = p_2$  for two-sample categorical data)
- 2. Calculate a Z-value using the sample data and an appropriate Normal model (ie:  $Z = \frac{(\hat{p}_1 - \hat{p}_2) - 0}{\sqrt{\frac{p_1(1-p_1)}{n} + \frac{p_2(1-p_2)}{n}}}$ )
- 3. Compare the Z-value to the Standard Normal curve to find the p-value
- 4. Use the p-value to make a conclusion (remember to consider context!)

# The Two-sample Z-test for Lister's Experiment

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- 2. We observed  $\hat{p}_1 \hat{p}_2 = 16/35 6/40 = 0.31$ , the pooled death rate is  $\hat{p} = 22/75 = 0.29$ , thus:  $Z = \frac{0.31 - 0}{\sqrt{0.29*(1 - 0.29)/35 + 0.29*(1 - 0.29)/40}} = 2.94$

$$Z = \frac{0.31 - 0}{\sqrt{0.29 * (1 - 0.29)/35 + 0.29 * (1 - 0.29)/40}} = 2.94$$

- 3. Using StatKey, a Z-value of 2.94 corresponds to a two-sided p-value of 0.0032
- 4. We conclude there is overwhelming statistical evidence that the new sterilization procedure leads to lower death rates



#### Effect Size

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- As we've previously discussed, hypothesis testing answers the question "could the observed difference be explained by random chance?"
  - ► This is a fundamentally different question from "is the observed difference large enough to change how we should act?"
- Confidence intervals are an important tool for answering the later question
  - ► The Normal model we've already presented makes for the easy construction of these interval estimates:

Point Estimate  $\pm$  Margin of Error

$$\hat{p}_1 - \hat{p}_2 \pm z^* \sqrt{\frac{\hat{p}_1(1-\hat{p}_1)}{n_1} + \frac{\hat{p}_2(1-\hat{p}_2)}{n_2}}$$



## Confidence Intervals for a Difference in Proportions

- One subtle difference when comparing the Normal model for confidence intervals with the one we used for hypothesis testing is the standard error
  - For hypothesis testing, the standard error used a *pooled* proportion to be consistent with the null hypothesis (which says  $p_1 = p_2$ )
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- ▶ Thus, for Lister's experiment we can 95% confident that between 11.1% and 50.9% more of a sterile surgery group will survive

$$\hat{p}_1 - \hat{p}_2 \pm z^* \sqrt{\frac{\hat{p}_1(1-\hat{p}_1)}{n_1} + \frac{\hat{p}_2(1-\hat{p}_2)}{n_2}}$$

$$0.46 - 0.15 \pm 1.96\sqrt{\frac{0.46(1 - 0.46)}{35} + \frac{0.15(1 - 0.15}{40}} = (0.111, 0.509)$$



## A Second Example

- Public health researchers often look at the proportion of a population who will develop a disease within a fixed time frame
  - Smoking is a well-established risk factor for lung cancer, but how do lung cancer rates compare among the populations of smokers and non-smokers?

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- Among all smokers, 0.44% are expected to develop lung cancer in a 10-year period
  - Among all non-smokers, only 0.05% are expected to develop lung cancer in a 10-year period
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- ► The difference in proportions is only 0.0039 (0.39%), so is it really worthwhile to get people to quit smoking?
  - ▶ I'd argue "yes", as the cancer risk is nearly 10 times higher!



#### Odds Ratios

- The most commonly reported measure of association describing the relationship between two categorical variables is the odds ratio
  - ► The *odds* of an event is the ratio of how often it happens to how often it doesn't happen
  - ▶ If a team has a 75% probability of winning a game, the odds of winning are 3, which is often spoken as "3 to 1"

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- In our smoking example, the odds of a smoker developing lung cancer are  $\frac{0.00438}{1-0.00438} = 0.00440$ 
  - Similarly, the odds of a non-smoker developing lung cancer are  $\frac{0.00045}{1-0.00045} = 0.00045$

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  - Similarly, the odds of a non-smoker developing lung cancer are  $\frac{0.00045}{1-0.00045} = 0.00045$
- ► Thus, the *odds ratio* is  $\frac{0.00440}{0.00045} = 9.8$ 
  - We say that the odds of a smoker developing lung cancer are 9.8 times those of a non-smoker developing lung cancer



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  - Instead, I only expect that you're able to properly interpret a CI estimate of an odds ratio
- ► For example, the 95% CI for the odds ratio in Lister's experiment is (1.4, 17.2)
  - ▶ This means the odds of dying in the control group are estimated (with 95% confidence) to be between 1.4 times and 17.2 times higher than the odds of dying in the sterile surgery group (quite an improvement!)

#### Conclusion

- This presentation introduced statistical methods for two-sample categorical data
  - Like one-sample categorical data, these methods are built upon a Normal model:

$$\hat{p}_1 - \hat{p}_2 \sim N\left(p_1 - p_2, \sqrt{\frac{p_1(1-p_1)}{n_1} + \frac{p_2(1-p_2)}{n_2}}\right)$$

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- When using this model for hypothesis testing, we use a pooled proportion to calculate the standard error
- ▶ When using this model for confidence interval estimation, we use the sample proportions to calculate the standard error