### STAT115: Introduction to Biostatistics

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## Lecture 27: Models for Binary Data

#### Outline

- Previous
  - Exploring (normal) models for continuous data
    - Single mean
    - Two independent groups
    - Paired data
    - Multiple independent groups
    - Linear regression
- Today
  - ► Consider data that are not continuous
  - Explore models for binary data

# Short Sighted

- Study from Australia following 1344 participants, aged 18-22.
- A total of 342 had myopia (-0.50D or worse defect)
- Assuming sample is representative, what can we learn at general prevalance of myopia in Australians in that age group?

#### **Problem**

- · We have been working with models for continuous outcome variables
- This is not continuous data
- It is binary data
  - ► Each observation is yes/no, success/failure, 1/0
  - ► Each participant either myopic ('success'), or not ('failure')
- Such data arises all the time
  - ▶ Will you support candidate X in the next election?
  - ▶ Did the chick successfully fledge?
  - ▶ Did the participant select option A (or B)?
  - ▶ Did the home team win the football match?
- We need a model for binary data
  - Probability distribution for binary data

### Bernoulli distribution

- Recall: discrete probability distributions
- Random variable Y with two possible outcomes: success/failure
  - ▶ Represent success with 1
  - Represent failure with 0
- These two outcomes have associated probabilities
  - ▶ Earlier in semester: we assigned them actual numbers, e.g. 0.6 and 0.4
  - ▶ Now: represent the probability of success with an (unknown) parameter: p
- That gives the probability distribution

| i              | 1   | 2 | Total |
|----------------|-----|---|-------|
| $y_i$          | 0   | 1 |       |
| $\Pr(Y = y_i)$ | 1-p | p | 1     |

### Bernoulli distribution: properties

Recall: we found means and variances of discrete probability distributions

$$E[Y] = \sum_{i=1}^{k} y_i \Pr(Y = y_i)$$

$$Var(Y) = \sum_{i=1}^{k} (y_i - E[Y])^2 \Pr(Y = y_i)$$

• Using these we can find the mean and variance of a Bernoulli distribution

$$E[Y] = p$$
 
$$\mathsf{Var}(Y) = p(1-p)$$

• Extension: Confirm these using the expectation and variance formulae above

# Binary to binomial

- Typically interested in cases where there are many binary trials
  - ▶ Flip a coin 15 times
  - Record the myopia status of 1344 individuals
- The number of successes from multiple trials has a binomial distribution, if:
  - 1. The trials are binary
    - The outcome can be represented as success / failure (or equivalent)
  - 2. The number of trials n, is fixed
    - e.g. the number of trials does not depend on the number of successes (or failures) you see
  - 3. The trials are independent
    - The outcome of one trial does not affect the outcome of another
  - 4. The probability of success, p, is the same for each trial
    - The probability of success does not change from one trial to another

# Binary to binomial

- Let's think about the simplest case
  - $ightharpoonup Y_1$  and  $Y_2$  are two (independent) random variables
  - ▶ Each of them has a Bernoulli distribution with probability of success p
- Our interest is in the random variable  $X=Y_1+Y_2$ 
  - Number of successes from two trials
- If we had a sample of 2 Australians (aged 18–22)
  - lacktriangleq X is a random variable that represents how many of them are myopic

#### Binomial distribution: n=2

• The probability distribution of  $X=Y_1+Y_2$  is

| i              | 1         | 2       | 3     | Total |
|----------------|-----------|---------|-------|-------|
| $x_i$          | 0         | 1       | 2     |       |
| $\Pr(X = x_i)$ | $(1-p)^2$ | 2p(1-p) | $p^2$ | 1     |

$$Pr(X=0) = \Pr(Y_1=0 \text{ and } Y_2=0)$$
 
$$= \Pr(Y_1=0) \Pr(Y_2=0) \qquad \text{multiplication rule: independence}$$
 
$$= (1-p) \times (1-p)$$

#### Binomial distribution: n=2

• The probability distribution of  $X = Y_1 + Y_2$  is

$$egin{array}{c|ccccc} i & 1 & 2 & 3 & {\sf Total} \\ \hline $x_i$ & 0 & 1 & 2 & \\ {\sf Pr}(X=x_i) & (1-p)^2 & 2p(1-p) & p^2 & 1 \\ \hline \end{array}$$

$$\Pr(X=1) = \Pr(Y_1=1 \text{ and } Y_2=0) + \Pr(Y_1=0 \text{ and } Y_2=1)$$
 
$$= \Pr(Y_1=1) \Pr(Y_2=0) + \Pr(Y_1=0) \Pr(Y_2=1) \qquad \text{independence}$$
 
$$= p(1-p) + (1-p)p$$

## Binomial distribution: general

- In general, the number of successes from n independent Bernoulli trials is:
  - $X = Y_1 + Y_2 + \ldots + Y_n$
- ullet For moderate or large values of n
  - ▶ Possible, but extremely tedious, to write out full probability distribution
- ullet We have a shortcut: we can find the probability of x successes from n independent Bernoulli trials

$$\Pr(X = x) = \binom{n}{x} p^x (1 - p)^{n - x}$$

### Binomial distribution: general

• The probability of x successes from n independent Bernoulli trials is

$$\Pr(X = x) = \binom{n}{x} p^x (1 - p)^{n - x}$$

- $\binom{n}{x} = \frac{n!}{x!(n-x)!}$  is the number of ways to obtain x successes from n trials<sup>1</sup>
- For each of these, the probability of observing those x successes is  $p^x(1-p)^{n-x}$ 
  - ▶ E.g. there are two ways to see x = 1 success from n = 2 trials (see above)
    - Each of those has probability p(1-p)
  - ▶ E.g. there are 3003 ways to see x=5 successes from n=15 trials
    - Each of these has probability  $p^5(1-p)^{10}$

 $x! = x \times (x-1) \times \ldots \times 3 \times 2 \times 1$ , e.g.  $3! = 3 \times 2 \times 1 = 6$ . x! is read as x factorial.

### Binomial distribution: general

• The probability of x successes from n independent Bernoulli trials is

$$\Pr(X = x) = \binom{n}{x} p^x (1 - p)^{n - x}$$

- We can use this to find the expectation and variance
  - ▶ The mean of a binomial distribution is E[X] = np
  - ▶ The variance of a binomial distribution Var(X) = np(1-p)
- If there are n=100 putts with probability of success p=0.2, then
  - $E[X] = np = 100 \times 0.2 = 20$
  - $ightharpoonup Var(X) = np(1-p) = 100 \times 0.2 \times 0.8 = 16$
  - $\blacktriangleright \ \operatorname{sd}(X) = \sqrt{\operatorname{Var}(X)} = 4$

## Binomial probabilities in R

- We don't have to calculate the long form of that equation
  - ▶ We can use the R function dbinom
- Example: what is Pr(X = 1) when p = 0.2 and n = 2

```
dbinom(x = 1, size = 2, prob = 0.2)
## [1] 0.32
```

- The arguments are:
  - $\triangleright$  x = 1: the number of successes x
  - $\triangleright$  size = 2: the number of trials n
  - ▶ prob = 0.2: the probability of success p
- Check that it gives the correct answer: we know it should be 2p(1-p)

```
2*0.2*(1-0.2)
## [1] 0.32
```

### More examples

- If we have sample of 15 individuals and probability myopia is 0.3 for each person:
- What is the probability that we see exactly 5 people in the sample with myopia?
- We have x = 5, n = 15, p = 0.3

```
dbinom(x = 5, size = 15, prob = 0.3)
## [1] 0.2061
```

• What is the probability of getting 40 myopic individuals out of sample of size 100 if p=0.35?

```
dbinom(x = 40, size = 100, prob = 0.35)
## [1] 0.04739
```

#### Back to the data

- We want to estimate the probability an Australia aged 18–22 is myopic
- What is our statistical model?
  - Myopia diagnosis individual eacd individual is a Bernoulli trial with probability p
    - Assume independence between individual results
  - ▶ Equivalently, the total number of successful putts is binomially distributed
- Want to estimate a parameter (population) with a statistic (sample)
  - (Reasonably) obvious statistic: sample proportion x/n
- For myopia data:

$$\hat{p} = \frac{x}{n} = \frac{342}{1344} = 0.254$$

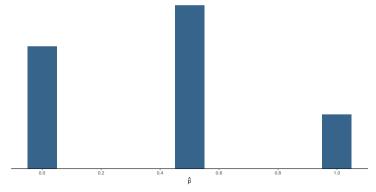
• Recall:  $\hat{p}$  is the estimate of parameter p

#### Confidence interval

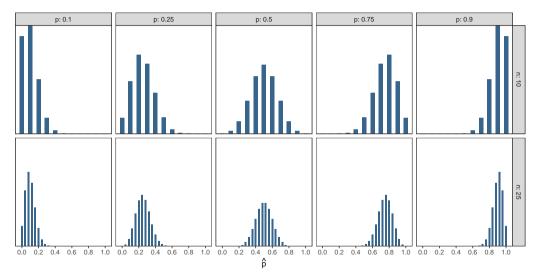
- How do we find a confidence interval?
- Recall: normal model
  - ► Found the sampling distribution
  - Obtained a confidence interval from the sampling distribution
- Can we do the same thing here?
  - ▶ The sampling distribution is the distribution of  $\hat{p}$  if we take repeated samples
- Look at it graphically

# Sampling distribution for $\hat{p}$ : Start small with n=2 and p=0.4

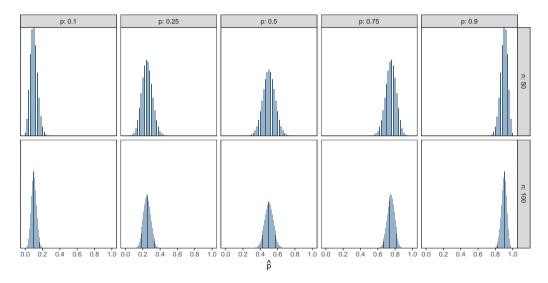
- There are three possibilities:
  - ▶ Observe x = 0 with probability 0.36: estimate  $\hat{p} = 0$
  - ▶ Observe x = 1 with probability 0.48: estimate  $\hat{p} = 0.5$
  - ▶ Observe x = 2 with probability 0.16: estimate  $\hat{p} = 1$



# Same principle, but increase the number of trials



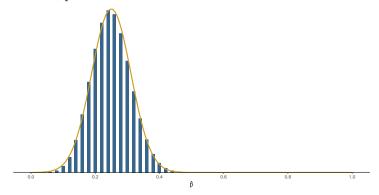
### Increase the number of trials some more



# Sampling distribution

- As the sample size gets larger, the sampling distribution looks increasingly normal
  - ► Normal pdf given in gold

• Example: n = 50, p = 0.25



# Sampling distribution

- We can approximate the sampling distribution by a normal distribution
  - Provided n is large enough
- There are various rules of thumb used to determine if the normal approximation is appropriate
- One of these is
  - ▶ np > 10 and n(1-p) > 10
- As we saw on the plots above, this reflects that
  - ightharpoonup The sampling distribution is increasingly normal as n increases
  - $\blacktriangleright$  When p is close to 0 or 1 it takes a larger n for it to approach normality
- In practice we use  $n\hat{p}$  and  $n(1-\hat{p})$  to check if a normal approximation is reasonable

# Sampling distribution

- We can approximate the sampling distribution by a normal distribution
  - Provided n is large enough
- The mean and variance are

$$E[\hat{p}] = p$$
 
$$\operatorname{Var}(\hat{p}) = \frac{p(1-p)}{n}$$

- So the standard error:  $\sigma_{\hat{p}} = \sqrt{\frac{p(1-p)}{n}}$
- Extension: Derive  $E[\hat{p}]$  and  $Var(\hat{p})$ 
  - ▶ We have  $\hat{P} = \frac{X}{n}$  where E[X] = np and Var(X) = np(1-p)

#### Confidence interval in R

• We use the normal approximation to find a confidence interval: prop.test

```
n = 1344; x = 342
prop.test(x, n)
##
    1-sample proportions test with continuity correction
##
## data: x out of n, null probability 0.5
## X-squared = 323, df = 1, p-value <2e-16
## alternative hypothesis: true p is not equal to 0.5
## 95 percent confidence interval:
    0.23154 0.27881
## sample estimates:
         g
## 0.25446
```

 We are 95% confident that the probability of myopia in a randomly sampled Australian aged 18-22 is between 0.232 and 0.279

# Hypothesis test

- We can also test the hypothesis
  - $ightharpoonup H_0: p = p_0$
  - $\vdash$   $\mathsf{H}_A: p \neq p_0$
- prop.test defaults to  $p_0 = 0.5$ 
  - ▶ It can be changed with option p, e.g. p = 0.2

# Hypothesis test

#### R output

```
n = 1344; x = 342
prop.test(x, n, p=0.2)
##
   1-sample proportions test with continuity correction
##
## data: x out of n, null probability 0.2
## X-squared = 25, df = 1, p-value = 7e-07
## alternative hypothesis: true p is not equal to 0.2
## 95 percent confidence interval:
## 0.232 0.279
## sample estimates:
##
      р
## 0.254
```

# Hypothesis test

#### continued

- Testing  $p_0=0.2$  (reflects myopia in 20-year olds in UK): gives a p-value of  $7\times 10^{-7}$
- This quantifies the incompatibility between the data and null hypothesis
- Since p-value  $< \alpha = 0.05$  there is (strong) evidence that the data are unusual given the null hypothesis is true
  - ► The data we have observed would be very unusual if the probability of myopia in Australians aged 18-22 was really 0.2.

### Summary

- Introduced Bernoulli distribution for binary observations
- The number of successes from multiple binary trials have binomial distribution
  - Several conditions need to be satisfied
- Use a binomial model to find:
  - ► Confidence interval for *p*
  - Hypothesis test
    - We will look more into these in the next lecture