MEDB 5501, Module 13

2024-11-12

Topics to be covered

- What you will learn
 - The two by two crosstabulaton
 - Test of equality of two proportions
 - Sample size calculations
 - R code
 - Your homework

```
data dictionary: titanic.txt
description: >
 The Titanic was a large cruise ship, the biggest of its kind in 1912. It was
 thought to be unsinkable, but when it set sail from England to America in
its
 maiden voyage, it struck an iceberg and sank, killing many of the passengers
 and crew. You can get fairly good data on the characteristics of passengers
 who died and compare them to those that survived. The data indicate a strong
 effect due to age and gender, representing a philosophy of "women and
children
 first" that held during the boarding of life boats.
additional description:
```

```
download_url:
    - http://www.statsci.org/data/general/titanic.txt

format:
    tab-delimited

varnames:
    first row of data

missing_value_code:
    NA
```

```
size:
  rows: 1313
  columns: 5

vars:
  Name:
    label: Passenger name

PClass:
    label: Passenger class
    scale: ordinal
    values: 1st, 2nd, 3rd
```

```
Age:
   unit: years
   scale: ratio
   range: positive real numbers
   missing: NA

Sex:
   scale: binary
   values: female, male
```

```
Survived:
scale: binary
values:
1: yes
0: no
```

The crosstabulation of two binary variables

```
Variable2
0 1
Variable1 0 n00 n01
1 n10 n11
```

Speaker notes

One of the most common tables you will see in Statistics is the 2 by 2 crosstabulation. This table shows the counts associated with the combination of the levels of the two binary variables. There are only four numbers in this table, but there are numerous statistics that you can use to summarize what's going on in this table.

Example: Titanic data

Crosstabulation

S	survi	ived
sex	yes	nc
female	308	154
male	142	709

Speaker notes

This is an example of a crosstabulation. The number in the upper left corner, 308, represents the number of female passengers who survived (did not die). This includes Kate Winslet. The number in the lower right corner, 709, represents then number of male passengers who did not survive. This includes, sad to say, Leonardo diCaprio.

Multiple ways to display, 1

Crosstabulation

	survi	ived
sex	yes	no
female	308	154
male	142	709

Swap rows

	survi	ived
sex	yes	no
male	142	709
female	308	154

Swap columns

	surv	ived
sex	no	yes
female	154	308
male	709	142

Swab both

```
survived sex no yes male 709 142 female 154 308
```

Speaker notes

The cross tabulation changes when you swap the rows, the columns, or both.

Multiple ways to display, 2

Transposed

sex
survived female male
yes 308 142
no 154 709

Transposed, swap rows

sex
survived female male
no 154 709
yes 308 142

Transposed, swap columns

sex
survived male female
yes 142 308
no 709 154

Transposed, swab both

sex
survived male female
no 709 154
yes 142 308

Speaker notes

You can get four more tables by transposing the matrix. What was the rows becomes the columns and what was the columns becomes the rows.

Row and column percents

Crosstabulation with row totals

survived sex yes no Sum female 308 154 462 male 142 709 851

Row percents

survived

sex yes no

Sum

female 0.6666667 0.3333333

1.0000000

male 0.1668625 0.8331375

1.0000000

 Cross tabulation with column totals

survived sex yes no female 308 154 male 142 709 Sum 450 863

Column percents

survived

sex yes no
female 0.6844444 0.1784473
male 0.3155556 0.8215527
Sum 1.0000000 1.0000000

Speaker notes

The column percents are computed by dividing by the column totals. They add up to 100% within each column. The row percents are computed by dividing by the row totals. They add up to 100% within each row. The cell percents are computed by dividing by the overall total. They only add up to 100% when you sum across both the rows and the columns.

Cell percents

Cell totals

survived sex yes no Sum female 308 154 462 male 142 709 851 Sum 450 863 1313

Cell percents

```
survived

sex yes no Sum

female 0.2345773 0.1172887 0.3518660

male 0.1081493 0.5399848 0.6481340

Sum 0.3427266 0.6572734 1.0000000
```

My recommendation

- Outcome variable is the columns
- Intervention/exposure variable is the rows
- Calculate row percentages

```
survived
sex yes no
female 0.6666667 0.3333333
male 0.1668625 0.8331375
```

Speaker notes

I have found that nine times out of ten, you want row percentages with the exposure/intervention variable as the rows and the outcome variable as the columns. This doesn't always work, but it is usually what I try first. It shows how much the chances of a good outcomes (or sometimes the chances of a bad outcome) change when you switch levels of the exposure/intervention.

The orientation is also important. You want the percentages that are most interesting as close as possible. This is the proximity principle. The values within a column are nested above/below the other. The values within a row are farther apart.

Always round, 1

```
0.6666667
- 0.1668625
-----
????????
```

Speaker notes Often in a cross tabulation, you will do some mental math. You might, for example, want to subtract survival probabilities.

Always round, 1

```
0.67
- 0.17
----
????
```

Speaker notes Notice how much easier the subtraction becomes when you round to two significant figures.

Break #1

- What you have learned
 - The two by two crosstabulaton
- What's coming next
 - Test of equality of two proportions

The binomial distribution

- You have n trials of an "experiment"
- Two outcomes, "success" or "failure"
- \(\pi\) is probability of success
- Each trial is independent

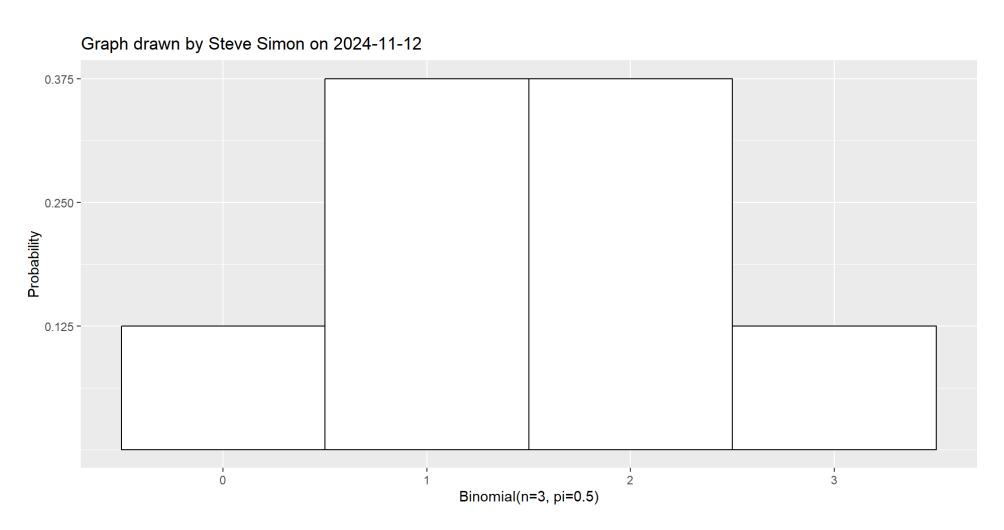
Example, creating a family of three children, 1

- You have 3 trials (pregnancies)
- Two outcomes, girl (success) or boy (failure)
- \(\pi\) = 0.5 is probability of success
- Each child is independent

Example, creating a family of three children, 2

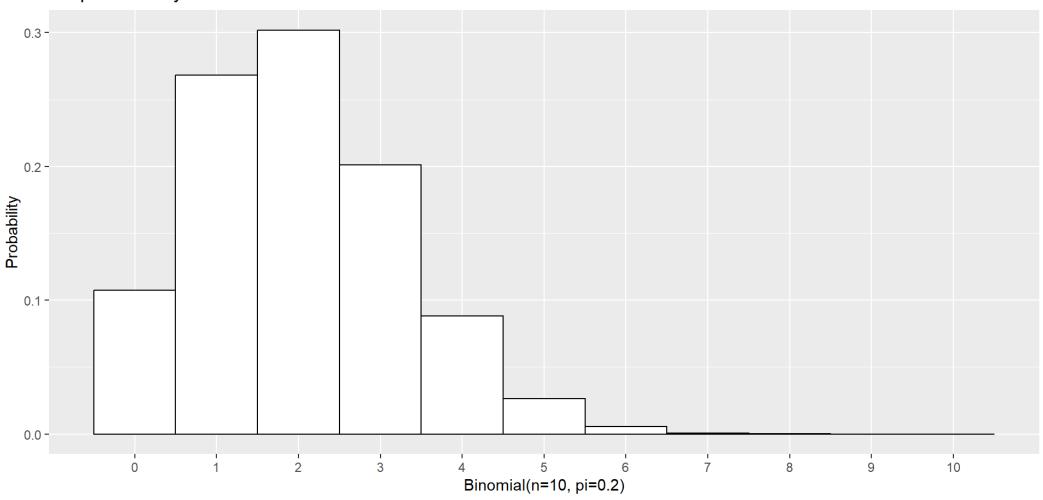
- Eight possible outcomes
 - X=0, BBB
 - X=1, BBG, BGB, GBB
 - X=2, BGG, GBG, GGB
 - X=3, GGG

Example, creating a family of three children, 3

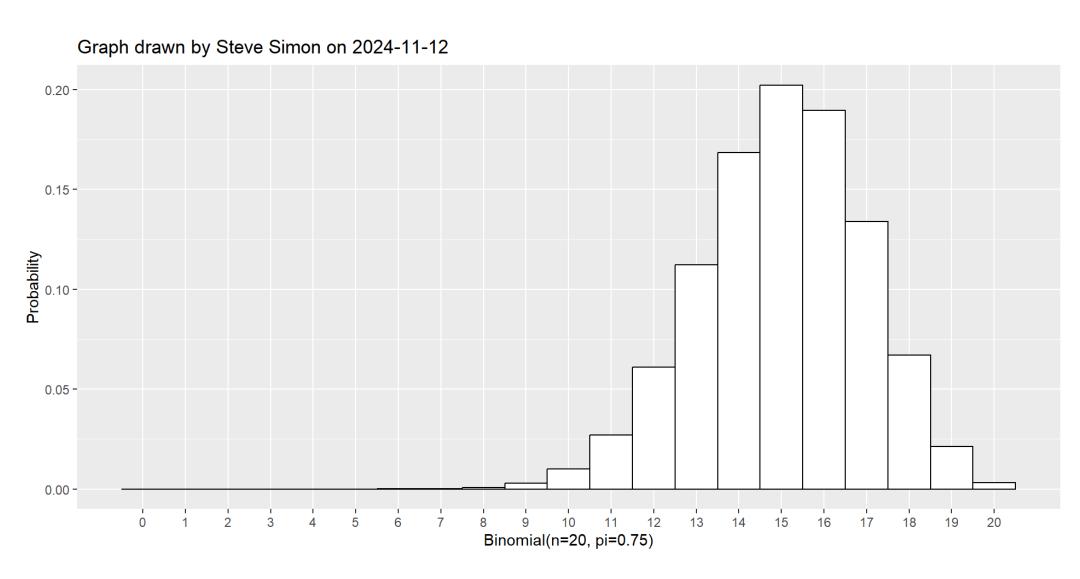


Example, Binomial(n=10, pi=0.2)





Example, Binomial(n=20, pi=0.75)



- Scenario 1
 - $\(X_1\)$ is a random binomial($\(n_1, \pi_1\)$)
 - $\(X_2\)$ is a random binomial($\(n_2, \pi_2\)$)

- Scenario 2
 - Sample \(X_{11}, X_{12}, ..., X_{1n_1}\)
 - Sample \(X_{21}, X_{22}, ..., X_{2n_2}\)
 - Only possible values are 0, 1
 - \(P[X_{1i}=1]=\pi_1\), \(P[X_{2i}=1]=\pi_2\)

- \(H_0:\\pi_1 \pi_2 = 0\)
- \(H_1:\\pi_1 \pi_2 \ne 0\)

- $\T = \frac{p_1-p_2}{se}$
 - \(se=\sqrt{\bar{p}(1-\bar{p})(\frac{1}{n_1}+\frac{1}{n_2})}\)
 - \(\bar{p}=\frac{n_1p_1+n_2p_2}{n_1+n_2}\)
- Accept \(H_0\) if T is close to zero.
 - If $\langle Z(\alpha/2) \rangle < T \langle Z(1-\alpha/2) \rangle$

- p-value = (2P[Z > |T|])
- Accept \(H_0\) if p-value > \(\alpha\)

Speaker notes

You can also compute a p-value which is the probability of observing the test statistic, T, or a value more extreme.

Some variations of the test statistic, 1

- Yates continuity correction
 - $\T = \frac{p_1-p_2 + c}{se}$
 - Formula for c is messy
 - Net effect is to pull T closer to zero
 - Better to meet the normal approximation

Some variations of the test statistic, 2

- Chi-squared test
 - $(T = \Big\{ (\frac{p_1-p_2}{se}\Big\} \Big)^2$
 - Accept \(H_0\) if T < \(\chi^2(1-\alpha, df=1)\)
 - p-value = \(P[\chi^2(df=1) > T]\)
 - Does not allow for easy test of one-sided hypothesis
- Chi-squared test with Yates continuity correction
 - $(T = \beta(\frac{p_1-p_2 + c}{se}\beta)^2)$

One-sided test, 1

- \(H_0:\\pi_1 \pi_2 = 0\)
- \(H_1:\\pi_1 \pi_2 > 0\)
 - Accept \(H_0\) if T < \(z(1-\alpha)\)
 - p-value = \(P[Z > T]\)
 - Accept \(H_0\) if p-value > \(\alpha\)

One-sided test, 2

- \(H_0:\\pi_1 \pi_2 = 0\)
- \(H_1:\\pi_1 \pi_2 < 0\)
 - Accept \(H_0\) if T > \(z(\alpha)\)
 - p-value = \(P[Z < T]\)</p>
 - Accept \(H_0\) if p-value > \(\alpha\)

Two sample test of proportions with Titanic data, 1

survived sex yes no female 308 154 male 142 709

Two sample test of proportions with Titanic data, 2

survived

sex yes no female 0.6666667 0.3333333 male 0.1668625 0.8331375

Two sample test of proportions with Titanic data, 3

2-sample test for equality of proportions without continuity correction

```
data: table1
X-squared = 332.06, df = 1, p-value < 2.2e-16
alternative hypothesis: two.sided
95 percent confidence interval:
    0.4500519 0.5495564
sample estimates:
    prop 1      prop 2
0.6666667 0.1668625</pre>
```

Break #2

- What you have learned
 - Test of equality of two proportions
- What's coming next
 - Sample size calculations

What you need for a continuous outcome

- Research hypothesis
- Standard deviation of your outcome measure
- Minimum clinically important difference
- Other details
 - Type I error rate (usually 0.05)
 - Power (usually 0.90)

Speaker notes

In an earlier lecture, you saw that a sample size calculation needed three major elements, a research hypothesis, a standard deviation, and the minimum clinically important difference.

That's not all, but the Type I error rate or alpha is usually fixed at 0.05. Power is usually fixed at 0.9. You might go a bit lower, but don't go much lower than 0.8 for power. It might be okay from your perspective, but power around 0.75 or less is considered a red flag by those who might examine your research (IRB members, granting agencies, journal peer reviewers).

What you need for a categorical outcome

- Research hypothesis
- Expected proportion in the control group
- Minimum clinically important difference

Speaker notes

The elements that you need when you have a categorical outcome change a bit. Instead of a standard deviation, you need to specify the expected proportion of events in the control group.

Hypothetical scenario, 1

- Weight loss study
 - Control is recommended diet and exercise routine
 - Treatment adds experimental weight loss drug
 - Binary event, losing at least 5 pounds after 6 months
- Hypothesis proportion is higher in treatment group than control
- Proportion in control group is 10%
- Need to see at least 5% more events in treatment group

Hypothetical scenario, 2

- Set power to 90%
- Set Type I error rate (alpha) to 5%

```
1 power.prop.test(
2    p1=0.10,
3    p2=0.15,
4    power=0.9,
5    sig.level=0.05)
```

Two-sample comparison of proportions power calculation

```
n = 917.3206
p1 = 0.1
p2 = 0.15
sig.level = 0.05
power = 0.9
alternative = two.sided
```

NOTE: n is number in *each* group

Rule of 50

- Want to detect a doubling or halving
- Need 25 to 50 events in each group
- Example
 - Control probability is 0.1
 - Want to see 20% in treatment group
 - = 25/0.1 = 250; 50/0.1 = 500

Break #3

- What you have learned
 - Sample size calculations
- What's coming next
 - R code

```
title: "Analysis of Titanic dataset"

format:
   html:
   embed-resources: true
---

This program reads data on survival of passengers on the Titanic. Find more information in the [data dictionary][web00].

[web00]: https://github.com/pmean/data/blob/main/files/titanic.yaml

This code was written by Steve Simon on 2024-11-09 and is placed in the public domain.
```

```
## Load the tidyverse library
```{r}
#| label: setup
#| message: false
#| warning: false
library(broom)
library(epitools)
library(tidyverse)
```
```

Comments on the code

For most of your programs, you should load the [tidyverse library] [web01]. The messages and warnings are suppressed.

[web01]: https://www.tidyverse.org/

In previous programs, I put a label for each chunk inside the curly braces ({}).

It is recommended instead to put the label on a separate line inside the program

chunk. It is a bit more work to provide a unique label for each chunk, but it helps quite a bit to isolate where to look when your code produces an error.

```
## Read the data and view a brief summary
```{r}
#| label: read
ti <- read_tsv(
 file="../data/titanic.txt",
 col_names=TRUE,
 col_types="ccncn",
 na="NA")
names(ti) <- tolower(names(ti))
glimpse(ti)
```</pre>
```

Comments on the code

Use read_tsv from the [readr package][web02] to read this file. Use col_names=TRUE because the column names are included as the first row of the file. The col_types="ccncn" specifies the first second and fourth columns as strings and the third and fifth as numeric. There are missing values in this dataset, designated by the letters "NA".

[web02]: https://readr.tidyverse.org/

```
## Replace numeric codes for survived
```{r}
#| label: replace-numbers
ti$survived <-
 factor(
 ti$survived,
 level=1:0,
 labels=c("yes", "no"))
```</pre>
```

```
#### Comments on the code
The [factor function] [web03] places the levels of a categorical variable in a
specific order and (optionally) attaches labels to each level. In this code,
the
number codes are reordered so that 1 appears first followed by 0. The labels
"yes" and "no" are attached to these two codes.
[web03]: https://stat.ethz.ch/R-manual/R-devel/library/base/html/factor.html
## Get counts of sex by survival
```{r}
| label: counts
table1 <-xtabs(~sex+survived, data=ti)
_ _ 1_ 7 _ 1
```

```
Comments on the code
```

The [table function] [web04] or the [xtabs function] [web05] creates a matrix with the number of observations in each combination of sex and survived. The [count function] [web06] provides an alternative where these values are placed in a single column.

```
[web04]: https://stat.ethz.ch/R-manual/R-devel/library/base/html/table.html
[web05]: https://stat.ethz.ch/R-manual/R-devel/library/stats/html/xtabs.html
[web06]: https://dplyr.tidyverse.org/reference/count.html
```

#### Interpretation of the output

There are 154 female passengers who died and 308 who survived. There are 709 male passengers who died and 142 who survived.

```
Get proportions for died/survived by sex
   ```{r}
#| label: proportions
table1 |>
   proportions("sex")
   ```
Comments on the code
```

The [proportions function] [pro1] calculate proportions for a matrix, either by row (margin=1), by column (margin=2) or across all cells (margin=NULL). This code shows an alternative where you specify which variable to summarize across. The line proportions ("sex") tells R to compute totals for each level of sex and divide by those totals. In this case, since sex represents the rows

#### Interpretation of the output

The proportion of women who died is 33%. The proportion of men who died is much higher at 83%.

```
Bar chart

```{r}

#| label: bar

ti |>
    ggplot() +
    aes(x = sex, fill = survived) +
    geom_bar(position = "fill") +
        xlab("Sex") +
        ylab("Proportion") +
        ggtitle("Graph drawn by Steve Simon on 2024-11-11")

*#### Comments on the code
```

```
#### Interpretation of the output
```

I am not a big fan of bar charts, but they are quite popular. This bar chart shows a large disparity in the proportion who survived in each sex (the upper portion of the bar in peach). Equivalently, you can say that there is a large disparity in the proportion who died in each sex (the lower portion of the bar in green-blue).

```
## Hypothesis test for two proportions
```{r}
#| label: equality-of-proportions
prop.test(table1, correct=FALSE)
```
```

Comments on the code

The [prop.test function][pro2] does not work on raw data. You need to get summary counts, either from the table function or the xtabs function. The option correct=FALSE informs R to not use the [Yates continuity correction] [yat1].

[pro2]: https://search.r-project.org/R/refmans/stats/html/prop.test.html
[yat1]: https://www.statskingdom.com/121proportion normal2.html

Interpretation of the output.

The Chi-squared statistic is much larger than the degrees of freedom and the p-value is small. So you would reject the null hypothesis and conclude that there is a statistically significant difference in the mortality rates between men and women on the Titanic. The 95% confidence interval for the difference in proportions is -0.55 to -0.45. This interval excludes the value of zero and indicates that the mortality rate is at least 45% lower and possibly as much as

55% lower for men.

```
## Power calculation
   ```{r}
#| label: power
power.prop.test(p1=0.17, p2=0.67, power=0.9)
   ```
```

```
#### Comments on the code
```

The [power.prop.test] [pow1] function will compute a sample size if you specify the two group probabilities, the significance level, and the power. It will also

do other calculations, such as the minimum difference in probabilities that you

could detect for a specific sample size and a specific power.

[pow1]: https://stat.ethz.ch/R-manual/Rdevel/library/stats/html/power.prop.test.html

Interpretation of the output

Clearly, a sample size of 1,313 passengers provided more than enough power and precision, especially considering that the difference in survival rates was so large. Out of curiosity, what sample size would still be adequate for testing a

shift of 50% from a survival rate of 17% to a survival rate of 67%? If you wanted power to be at least 90%, then a sample size of only 19 passengers of each sex would be needed.

```
## Chi-squared test
   ```{r}
#| label: chi-square-test
chisq.test(table1, correct=FALSE)
   ```
```

```
#### Comments on the code
```

The [chisq.test function][chi1] calculates a chi-square test of independence. It.

takes input in a variety of forms. In this example, it uses a crosstabulation computed by the xtabs command as input.

This function also will run a goodness-of-fit test, which is not discussed in this lecture.

[chi1]: https://stat.ethz.ch/R-manual/Rdevel/library/stats/html/chisq.test.html

```
#### Interpretation of the output
The chi-squared statistic is much larger than the degrees of freedom and the
p-value is small. You should reject the null hypothesis and conclude that sex
and survival are related (not independent)

## Fisher's Exact test

```{r}
#| label: fishers-exact
fisher.test(table1)
```
```

Comments on the code

The [fisher.test function][fis1] calculates the Fisher's exact test, which is helpful for small sample sizes. The 1,313 passengers on the Titanic do not constitute a small sample size by any means. This test is just shown as an example of how to calculate this test.

[fis1]: https://stat.ethz.ch/R-manual/Rdevel/library/stats/html/fisher.test.html

```
#### Interpretation of the output
The p-value is small. You should reject the null hypothesis and conclude that
sex and survival are related (not independent). The estimated odds ratio is
9.97. The confidence interval for the odds ratio excludes the value of 1
leading to the same conclusion. In fact, even after allowing for sampling
error
that odds of survival are at least 7.6 times greater for women than for me.

## Odds ratio calculation

```{r}
#| label: odds-ratio
oddsratio(table1)
```
```

Comments on the code

The oddsratio function and riskratio function (see below) are part of the [epitools library][epi1]. It produces an odds ratio and confidence interval and p-values associated with the Fisher's Exact test and the Chi-squared test of independence.

[epi1]: https://cran.r-project.org/web/packages/epitools/epitools.pdf

Interpretation of the output

We are 95% confident that the odds ratio of survival for women versus men is at.

least 7.7 and possibly as large as 13, after accounting for sampling error. This interval excludes the value of 1, so you can conclude that the risk of death is much higher for men than for women. Equivalently you could conclude that the odds of survival are much higher for women than for men.

```
## Risk ratio calculation, 1

```{r}
#| label: risk-ratio-1
table1 |>
 proportions("sex")

Interpretation of the output
```

Before calculating the risk ratio, let's look at the row percentages one more time. The probability of survival is around 2/3 for women and about 1/6 for men. This means that the risk ratio from a survival perspective is around 4 (2/3 divided by 1/6). The probability of death is 1/3 for females and about 5/6 for males. The risk ratio from a mortality perspective is 0.4 (1/3 divided by 5/6).

```
Risk ratio calculation, 2

```{r}

#| label: risk-ratio-2
riskratio(table1)

.``

#### Interpretation of the output

The risk ratio is comparing the probability of death between m
```

The risk ratio is comparing the probability of death between men and women. Men have 2.5 times higher probability of death compared to women. The confidence interval excludes the value of 1, indicating a statistically significant increase.

```
## Risk ratio calculation, 3

```{r}
#| label: risk-ratio-3
riskratio(table1, rev="columns")

Interpretation of the output
```

The risk ratio is comparing the probability of survival between men and women. Men has one-fourth the probability of survival compared to women. The confidence interval excludes the value of 1, indicating that men have a statistically significantly lower probability of survival compared to women.

```
Save data for later use

```{r save}
save(ti, file="../data/titanic.RData")

#### Comments on the code

It is usually a good idea to [save][sav1] your data in an RData file to make it
easier to retrieve this data later (with the [load function][loa1]).

[sav1]: https://stat.ethz.ch/R-manual/R-devel/library/base/html/save.html
[loa1]: https://stat.ethz.ch/R-manual/R-devel/library/base/html/save.html
```

Break #4

- What you have learned
 - R code
- What's coming next
 - Your homework

title: "Directions for 5501-13 programming assignment"

This programming assignment was written by Steve Simon on 2024-10-08 and is placed in the public domain.

```
## Program
```

- Download the [program] [tem]
 - Store it in your src folder
- Modify the file name
 - Use your last name instead of "simon"
- Modify the documentation header
 - Add your name to the author field
 - Optional: change the copyright statement

[tem]: https://github.com/pmean/classes/blob/master/biostats-1/13/src/simon-5501-13-titanic.qmd

Data

```
- Download the [data][dat] file
- Store it in your data folder
- Refer to the [data dictionary][dic], if needed.

[dat]: https://github.com/pmean/data/blob/main/files/titanic.txt
[dic]: https://github.com/pmean/data/blob/main/files/titanic.yaml
```

```
## Question 1
Create a new variable, third_class that indicates whether a passenger is in third class or not. The code would look something like this.

```{}
ti$third_class <-
 case_when(
 ti$pclass == "1st" ~ "no",
 ti$pclass == "2nd" ~ "no",
 ti$pclass == "3rd" ~ "yes")

How many passengers were in third class?</pre>
```

```
Question 2
```

What are the probabilities of survival for third class passengers. How does this

compare to the probability of survival for the other passengers.

## Question 3

Test the hypothesis that the survival probability is different for third class passengers and the other passengers. Interpret the p-value and confidence interval.

```
Your submission
 Save the output in html format
 Convert it to pdf format.
 Make sure that the pdf file includes
 - Your last name
 - The number of this course
 - The number of this module
 Upload the file
If it doesn't work
Please review the [suggestions if you encounter an error page][sim3].
[sim3]: https://github.com/pmean/classes/blob/master/general/suggestions-if-
```

### Summary

- What you have learned
  - The two by two crosstabulaton
  - Test of equality of two proportions
  - Sample size calculations
  - R code
  - Your homework