

# Data Science for Biological, Medical and Health Research: Notes for 431

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# Introduction

These Notes provide a series of examples using R to work through issues that are likely to come up in PQHS/CRSP/MPHP 431.

While these Notes share some of the features of a textbook, they are neither comprehensive nor completely original. The main purpose is to give 431 students a set of common materials on which to draw during the course. In class, we will sometimes:

- reiterate points made in this document,
- amplify what is here,
- simplify the presentation of things done here,
- use new examples to show some of the same techniques,
- refer to issues not mentioned in this document,

but what we don't do is follow these notes very precisely. We assume instead that you will read the materials and try to learn from them, just as you will attend classes and try to learn from them. We welcome feedback of all kinds on this document or anything else. Just email us at 431-help at case dot edu, or submit a pull request.

What you will mostly find are brief explanations of a key idea or summary, accompanied (most of the time) by R code and a demonstration of the results of applying that code.

Everything you see here is available to you as HTML or PDF. You will also have access to the R Markdown files, which contain the code which generates everything in the document, including all of the R results. We will demonstrate the use of R Markdown (this document is generated with the additional help of an R package called `bookdown`) and R Studio (the “program” which we use to interface with the R language) in class.

To download the data and R code related to these notes, visit <https://github.com/THOMASELOVE/431data>

## Structure

The Notes, like the 431 course, fall in three main parts.

Part A is about **visualizing data and exploratory data analyses**. These Notes focus on using R to work through issues that arise in the process of exploring data, managing (cleaning and manipulating) data into a tidy format to facilitate useful work downstream, and describing those data effectively with visualizations, numerical summaries, and some simple models.

Part B is about **making comparisons** with data. The Notes discuss the use of R to address comparisons of means and of rates/proportions, primarily. The main ideas include confidence intervals, the bootstrap and parametric and non-parametric tests of hypotheses. Key ideas from Part A that have an impact here include visualizations to check the assumptions behind our inferences, and cleaning/manipulating data to facilitate our comparisons.

Part C is about **building models** with data. The Notes are primarily concerned (in 431) with linear regression models for continuous quantitative outcomes, using one or more predictors. We'll see how to use

models to accomplish many of the comparisons discussed in Part B, and make heavy use of visualization and data management tools developed in Part A to assess our models.

## Course Philosophy

In developing this course, we adopt a modern approach that places data at the center of our work. Our goal is to teach you how to do truly reproducible research with modern tools. We want you to be able to answer real questions using data and equip you with the tools you need in order to answer those questions well (Çetinkaya-Rundel (2017) has more on a related teaching philosophy.)

The curriculum includes more on several topics than you might expect from a standard graduate introduction to statistics.

- data gathering
- data wrangling
- exploratory data analysis and visualization
- multivariate modeling
- communication

It also nearly completely avoids formalism and is extremely applied - this is most definitely **not** a course in theoretical or mathematical statistics.

The 431 course is about **getting things done**. It's not a statistics course, nor is it a computer science course. It is instead a course in **data science**.

# Chapter 1

## Data Science

The definition of **data science** can be a little slippery. One current view of data science, is exemplified by Steven Geringer's 2014 Venn diagram.

- The field encompasses ideas from mathematics and statistics and from computer science, but with a heavy reliance on subject-matter knowledge. In our case, this includes clinical, health-related, medical or biological knowledge.
- As Gelman and Nolan (2017) suggest, the experience and intuition necessary for good statistical practice are hard to obtain, and teaching data science provides an excellent opportunity to reinforce statistical thinking skills across the full cycle of a data analysis project.
- The principal form in which computer science (coding/programming) play a role in this course is to provide a form of communication. You'll need to learn how to express your ideas not just orally and in writing, but also through your code.

### 1.1 Why a unicorn?

Data Science is a **team** activity. Everyone working in data science brings some part of the necessary skillset, but no one person can cover all three areas alone for excellent projects.

[The individual who is truly expert in all three key areas (mathematics/statistics, computer science and subject-matter knowledge) is] a mythical beast with magical powers who's rumored to exist but is never actually seen in the wild.

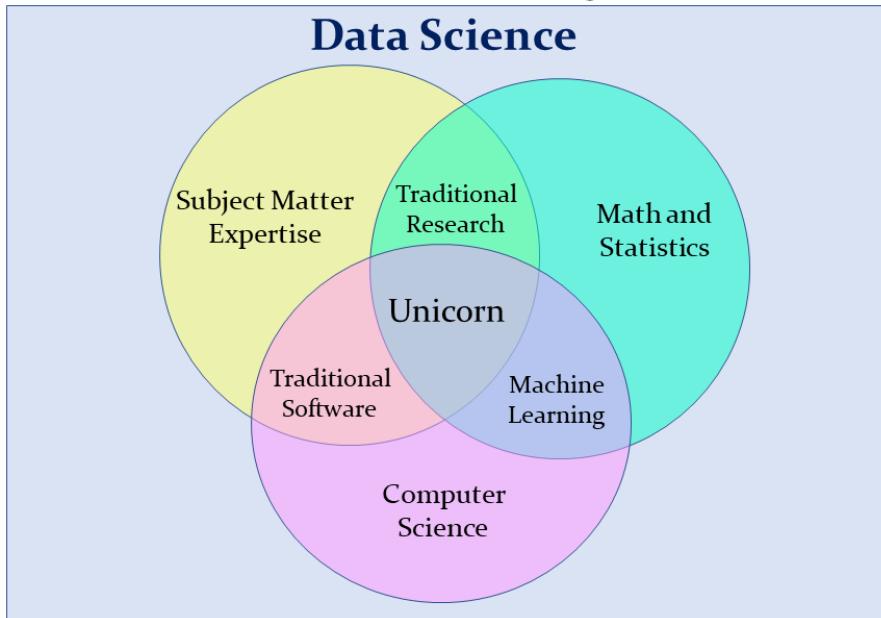
<http://www.kdnuggets.com/2016/10/battle-data-science-venn-diagrams.html>

### 1.2 Data Science Project Cycle

A typical data science project can be modeled as follows, which comes from the introduction to the amazing book **R for Data Science**, by Garrett Grolemund and Hadley Wickham, which is a key text for this course (Grolemund and Wickham 2017).

This diagram is sometimes referred to as the Krebs Cycle of Data Science. For more on the steps of a data science project, we encourage you to read the Introduction of Grolemund and Wickham (2017).

## Data Science Venn Diagram 2.0



Original Image Copyright © 2014 by Steven Geringer, Raleigh NC.  
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Figure 1.1: Data Science Venn Diagram from Steven Geringer

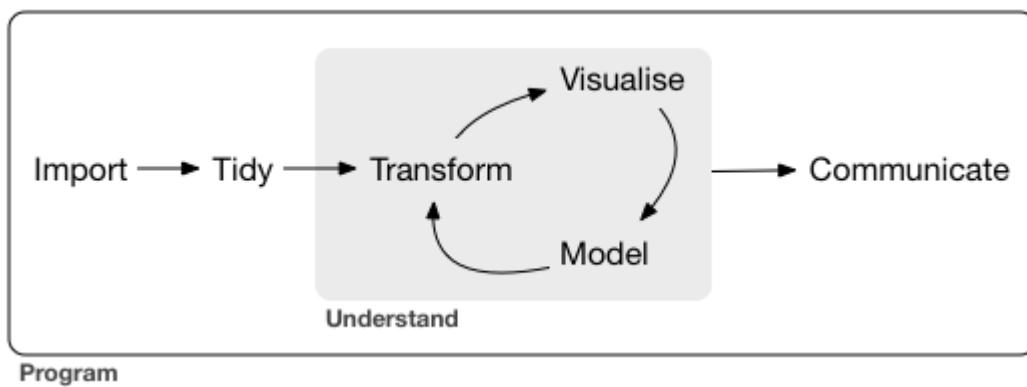


Figure 1.2: Source: R for Data Science: Introduction

## 1.3 What Will We Discuss in 431?

We'll discuss each of these elements in the 431 course, focusing at the start on understanding our data through transformation, modeling and (especially in the early stages) visualization. In 431, we learn how to get things done.

- We get people working with R and R Studio and R Markdown, even if they are completely new to coding. A gentle introduction is provided at Ismay and Kim (2017)
- We learn how to use the `tidyverse` (<http://www.tidyverse.org/>), an array of tools in R (mostly developed by Hadley Wickham and his colleagues at R Studio) which share an underlying philosophy to make data science faster, easier, more reproducible and more fun. A critical text for understanding the tidyverse is Grolemund and Wickham (2017). Tidyverse tools facilitate:
  - **importing** data into R, which can be the source of intense pain for some things, but is really quite easy 95% of the time with the right tool.
  - **tidying** data, that is, storing it in a format that includes one row per observation and one column per variable. This is harder, and more important, than you might think.
  - **transforming** data, perhaps by identifying specific subgroups of interest, creating new variables based on existing ones, or calculating summaries.
  - **visualizing** data to generate actual knowledge and identify questions about the data - this is an area where R really shines, and we'll start with it in class.
  - **modeling** data, taking the approach that modeling is complementary to visualization, and allows us to answer questions that visualization helps us identify.
  - and last, but definitely not least, **communicating** results, models and visualizations to others, in a way that is reproducible and effective.
- Some programming/coding is an inevitable requirement to accomplish all of these aims. If you are leery of coding, you'll need to get past that, with the help of this course and our stellar teaching assistants. Getting started is always the most challenging part, but our experience is that most of the pain of developing these new skills evaporates by early October.
- Having completed some fundamental work in Part A of the course, we then learn how to use a variety of R packages and statistical methods to accomplish specific inferential tasks (in Part B, mostly) and modeling tasks (in Part C, mostly.)



# Chapter 2

## Setting Up R

These Notes make extensive use of

- the statistical software language R, and
- the development environment R Studio,

both of which are free, and you'll need to install them on your machine. Instructions for doing so are in found in the course syllabus.

If you need an even gentler introduction, or if you're just new to R and RStudio and need to learn about them, we encourage you to take a look at <http://moderndive.com/>, which provides an introduction to statistical and data sciences via R at Ismay and Kim (2017).

### 2.1 R Markdown

These notes were written using R Markdown. R Markdown, like R and R Studio, is free and open source.

R Markdown is described as an *authoring framework* for data science, which lets you

- save and execute R code
- generate high-quality reports that can be shared with an audience

This description comes from <http://rmarkdown.rstudio.com/lesson-1.html> which you can visit to get an overview and quick tour of what's possible with R Markdown.

Another excellent resource to learn more about R Markdown tools is the Communicate section (especially the R Markdown chapter) of Grolemund and Wickham (2017).

### 2.2 R Packages

To start, I'll present a series of commands I run at the beginning of these Notes. These particular commands set up the output so it will look nice as either an HTML or PDF file, and also set up R to use several packages (libraries) of functions that expand its capabilities. A chunk of code like this will occur near the top of any R Markdown work.

```
knitr::opts_chunk$set(comment = NA)

library(boot); library(devtools); library(forcats)
library(grid); library(knitr); library(pander)
```

```
library(pwr); library(viridis); library(NHANES)
library(tidyverse)

source("data/Love-boost.R")
```

I have deliberately set up this list of loaded packages/libraries to be relatively small, and will add some other packages later, as needed. You only need to install a package once, but you need to reload it every time you start a new session.

## 2.3 Other Packages

I will also make use of functions in the following packages/libraries, but when I do so, I will explicitly specify the package name, using a command like `Hmisc::describe(x)`, rather than just `describe(x)`, so as to specify that I want the Hmisc package's version of `describe` applied to whatever `x` is. Those packages are:

- `aplypack` which provides `stem.leaf` and `stem.leaf.backback` for building fancier stem-and-leaf displays
- `arm` which provides a set of functions for model building and checking that are used in Gelman and Hill (2007)
- `broom` which turns the results lots of different analyses in R into more useful tidy data frames (tibbles.)
- `car` which provides some tools for building scatterplot matrices, but also many other functions described in Fox and Weisberg (2011)
- `cowplot` which is used in Part C to put multiple graphical objects in the same plot, like `gridExtra`: <https://cran.r-project.org/web/packages/cowplot/vignettes/introduction.html>
- `Epi` for 2x2 table analyses and materials for classical epidemiology: <http://BendixCarstensen.com/Epi/>
- `GGally` for scatterplot and correlation matrix visualizations: <http://ggobi.github.io/ggally/>
- `ggridges` which is used to make ridgeline plots
- `gridExtra` which includes a variety of functions for manipulating graphs: <https://github.com/baptiste/gridextra>
- `Hmisc` from Frank Harrell at Vanderbilt U., for its version of `describe` and for many regression modeling functions we'll use in 432. Details on Hmisc are at <http://biostat.mc.vanderbilt.edu/wiki/Main/Hmisc>. Frank has written several books - the most useful of which for 431 students is probably Harrell and Slaughter (2017)
- `mice`, which we'll use (a little) in 431 for multiple imputation to deal with missing data: <http://www.stefvanbuuren.nl/mi/>
- `mosaic`, mostly for its `favstats` summary, but Project MOSAIC is a community of educators you might be interested in: <http://mosaic-web.org/>
- `psych` for its own version of `describe`, but other features are described at <http://personality-project.org/r/psych/>

We also will use a package called `xda` for two functions called `numSummary` and `charSummary`, but that package gets loaded via `devtools` and GitHub by the code in these Notes.

Several other packages are included below, even though they are not used in these Notes, because they will be used in class sessions or in 432.

When compiling the Notes from the original code files, these packages will need to be installed (but not loaded) in R, or an error will be thrown when compiling this document. To install all of the packages used within these Notes, type in (or copy and paste) the following commands and run them in the R Console. Again, you only need to install a package once, but you need to reload it every time you start a new session.

```
pkgs <- c("aplypack", "arm", "babynames", "boot", "broom", "car", "cowplot",
"devtools", "Epi", "faraway", "forcats", "foreign", "gapminder",
"GGally", "ggridges", "gridExtra", "Hmisc", "knitr", "lme4", "magrittr",
"markdown", "MASS", "mice", "mosaic", "multcomp", "NHANES",
"pander", "psych", "pwr", "qcc", "rmarkdown", "rms", "sandwich",
```

```
"survival", "tableone", "tidyverse", "vcd", "viridis")  
install.packages(pkgs)
```



## Part A. Exploring Data



# Chapter 3

## Visualizing Data

Part A of these Notes is designed to ease your transition into working effectively with data, so that you can better understand it. We'll start by visualizing some data from the US National Health and Nutrition Examination Survey, or NHANES. We'll display R code as we go, but we'll return to all of the key coding ideas involved later in the Notes.

### 3.1 The NHANES data: Collecting a Sample

To begin, we'll gather a random sample of 1,000 subjects participating in NHANES, and then identify several variables of interest about those subjects<sup>1</sup>. The motivation for this example came from a Figure in Baumer, Kaplan, and Horton (2017).

```
# library(NHANES) # already loaded NHANES package/library of functions, data  
  
set.seed(431001)  
# use set.seed to ensure that we all get the same random sample  
# of 1,000 NHANES subjects in our nh_data collection  
  
nh_data <- sample_n(NHANES, size = 1000) %>%  
  select(ID, Gender, Age, Height, Weight, BMI, Pulse, Race1, HealthGen, Diabetes)  
  
nh_data  
  
# A tibble: 1,000 x 10  
  ID   Gender  Age  Height  Weight    BMI Pulse    Race1 HealthGen  
  <int> <fctr> <int>  <dbl>   <dbl>  <dbl> <int> <fctr>   <fctr>  
1 59640   male    54    176  129.0  41.8     74  White    Good  
2 59826 female    67    156   50.2  20.5     66  White    Vgood  
3 56340   male     9    128   23.3  14.2     86  Black    <NA>  
4 56747   male    33    194  105.1  27.9     68  White    Vgood  
5 51754 female    58    167  106.0  37.9     70  White    <NA>  
6 52712   male     6    109   16.9  14.3     NA  White    <NA>  
7 63908   male    55    169   90.6  31.9     62  Mexican  Vgood  
8 60865 female    25    156   55.0  22.8     58  Other    Vgood  
9 66642   male    41    178   89.3  28.2     72  White    Vgood  
10 59880  female   45    163   98.3  36.9     80 Hispanic Good
```

<sup>1</sup>For more on the NHANES data available in the NHANES package, type ?NHANES in the Console in R Studio.

```
# ... with 990 more rows, and 1 more variables: Diabetes <fctr>
```

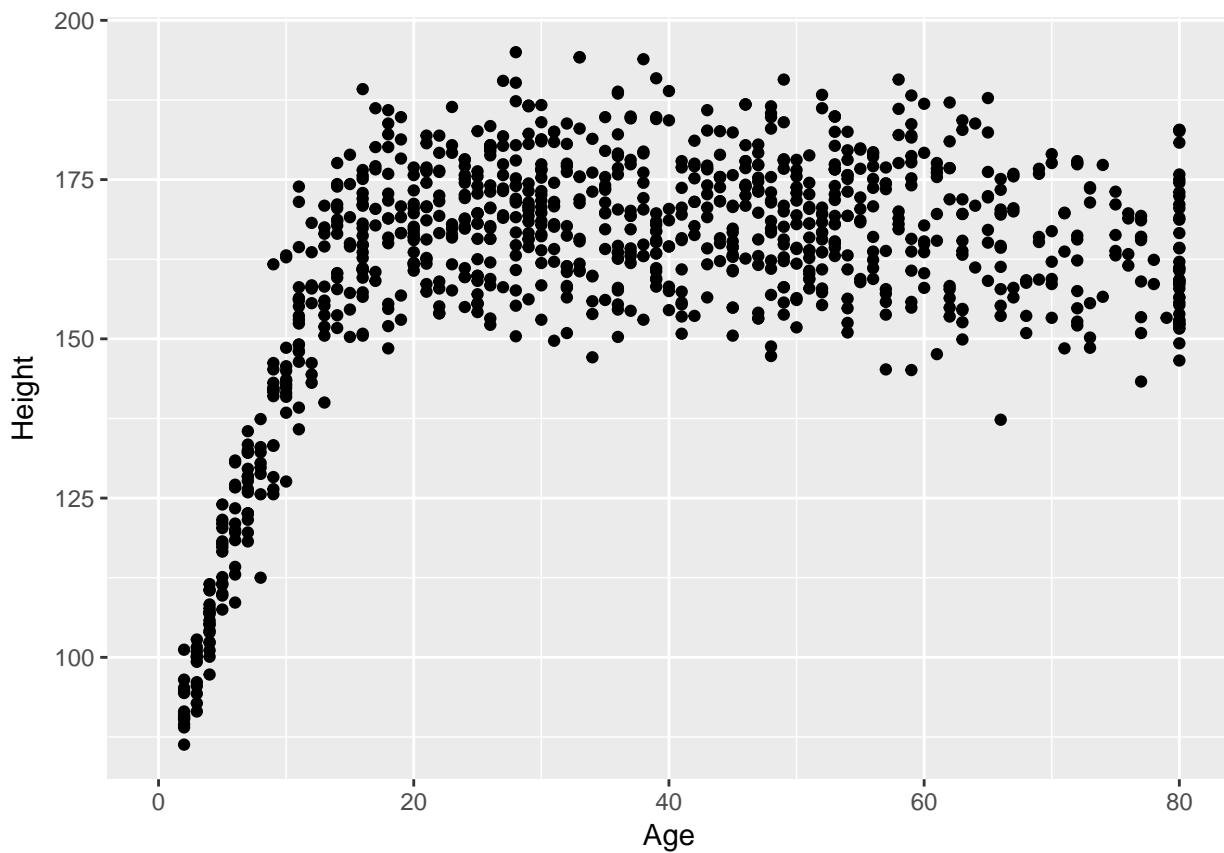
We have 1000 rows (observations) and 10 columns (variables) that describe the subjects listed in the rows.

## 3.2 Age and Height

Suppose we want to visualize the relationship of Height and Age in our 1,000 NHANES observations. The best choice is likely to be a scatterplot.

```
ggplot(data = nh_data, aes(x = Age, y = Height)) +
  geom_point()
```

Warning: Removed 25 rows containing missing values (geom\_point).



We note several interesting results here.

1. As a warning, R tells us that it has “Removed 25 rows containing missing values (geom\_point).” Only 975 subjects plotted here, because the remaining 25 people have missing (NA) values for either Height, Age or both.
2. Unsurprisingly, the measured Heights of subjects grow from Age 0 to Age 20 or so, and we see that a typical Height increases rapidly across these Ages. The middle of the distribution at later Ages is pretty consistent at a Height somewhere between 150 and 175. The units aren’t specified, but we expect they must be centimeters. The Ages are clearly reported in Years.
3. No Age is reported over 80, and it appears that there is a large cluster of Ages at 80. This may be due to a requirement that Ages 80 and above be reported at 80 so as to help mask the identity of those

individuals.<sup>2</sup>

As in this case, we're going to build most of our visualizations using tools from the `ggplot2` package, which is part of the `tidyverse` series of packages. You'll see similar coding structures throughout this Chapter, most of which are covered as well in Chapter 3 of Grolmund and Wickham (2017).

### 3.3 Subset of Subjects with Known Age and Height

Before we move on, let's manipulate the data set a bit, to focus on only those subjects who have complete data on both Age and Height. This will help us avoid that warning message.

```
nh_dat2 <- nh_data %>%
  filter(complete.cases(Age, Height))

summary(nh_dat2)

      ID      Gender       Age      Height
Min. :51654  female:498  Min.   : 2.0  Min.   :86.3
1st Qu.:56752  male   :477   1st Qu.:20.0  1st Qu.:156.4
Median :61453                    Median :36.0  Median :165.8
Mean   :61602                    Mean   :37.3  Mean   :161.7
3rd Qu.:66484                   3rd Qu.:53.0  3rd Qu.:174.1
Max.   :71826                   Max.   :80.0  Max.   :195.0

      Weight      BMI      Pulse      Race1
Min.   : 12.5  Min.   :13.2  Min.   : 42.0  Black   :112
1st Qu.: 57.6  1st Qu.:21.6  1st Qu.: 66.0  Hispanic: 69
Median : 73.4  Median :26.1  Median : 72.0  Mexican :104
Mean   : 73.4  Mean   :27.0  Mean   : 73.7  White   :607
3rd Qu.: 90.2  3rd Qu.:31.1  3rd Qu.: 82.0  Other   : 83
Max.   :198.7  Max.   :80.6  Max.   :124.0
NA's   : 2      NA's   : 2    NA's   :120

      HealthGen Diabetes
Excellent: 87 No     :910
Vgood   :276 Yes    : 64
Good    :276 NA's  : 1
Fair    :103
Poor    : 15
NA's   :218
```

Note that the units and explanations for these variables are contained in the NHANES help file, available via `?NHANES` in the Console of R Studio.

### 3.4 Age-Height and Gender?

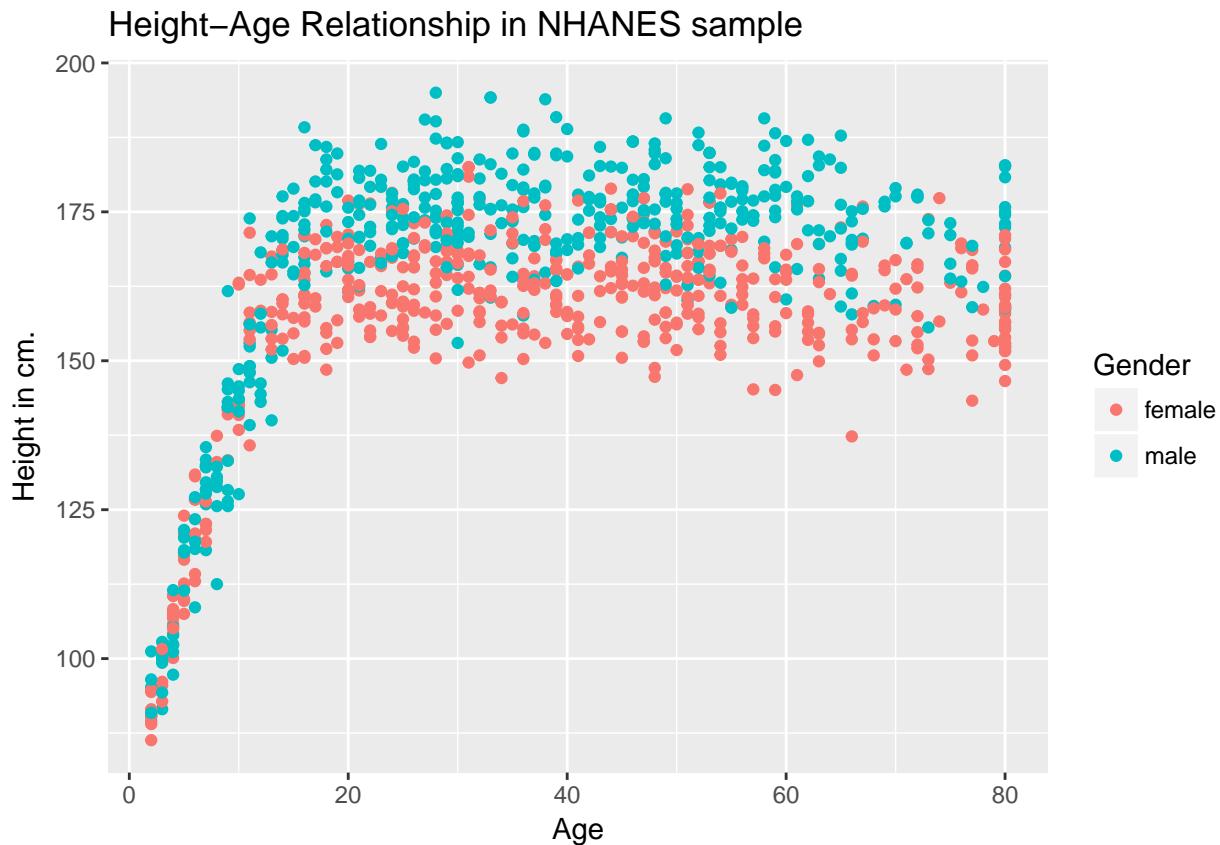
Let's add Gender to the plot using color, and also adjust the y axis label to incorporate the units of measurement.

```
ggplot(data = nh_dat2, aes(x = Age, y = Height, color = Gender)) +
  geom_point()
```

---

<sup>2</sup>If you visit the NHANES help file with `?NHANES`, you will see that subjects 80 years or older were indeed recorded as 80.

```
labs(title = "Height-Age Relationship in NHANES sample",
     y = "Height in cm.")
```

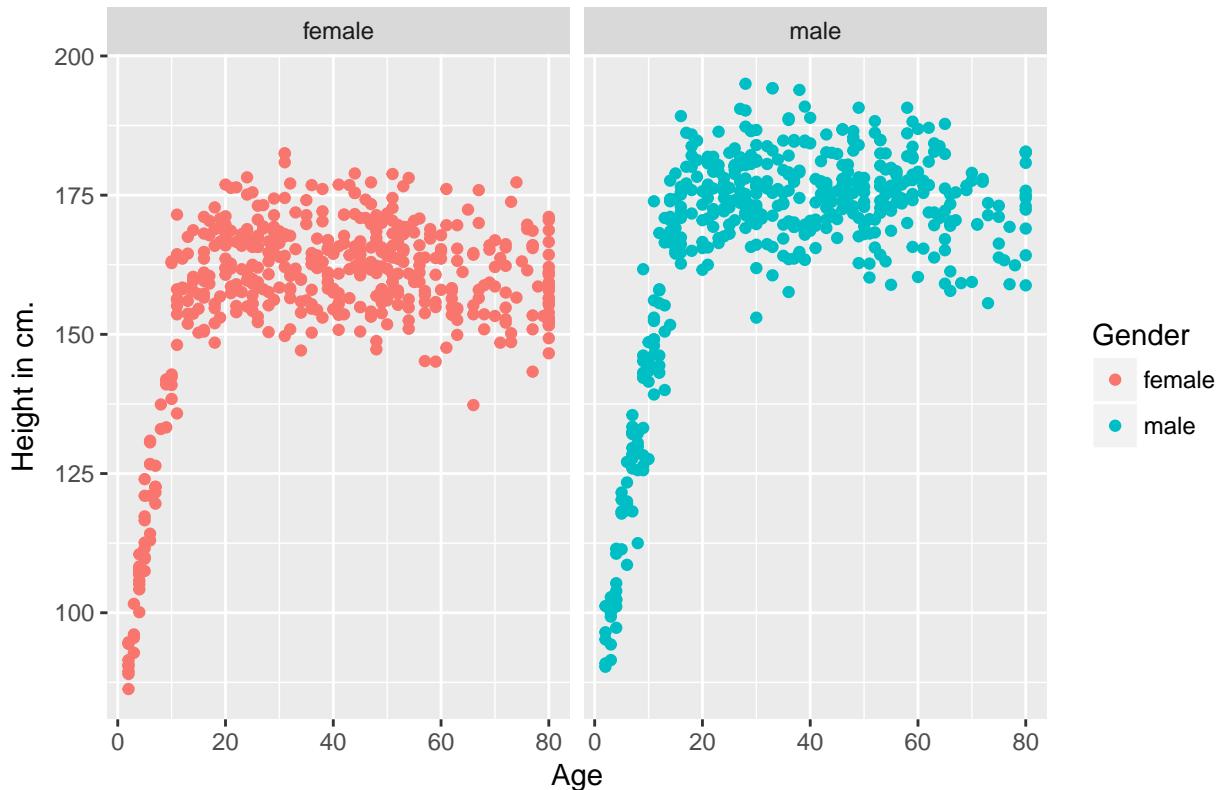


### 3.4.1 Can we show the Female and Male relationships in separate panels?

Sure.

```
ggplot(data = nh_dat2, aes(x = Age, y = Height, color = Gender)) +
  geom_point() +
  labs(title = "Height-Age Relationship in NHANES sample",
       y = "Height in cm.") +
  facet_wrap(~ Gender)
```

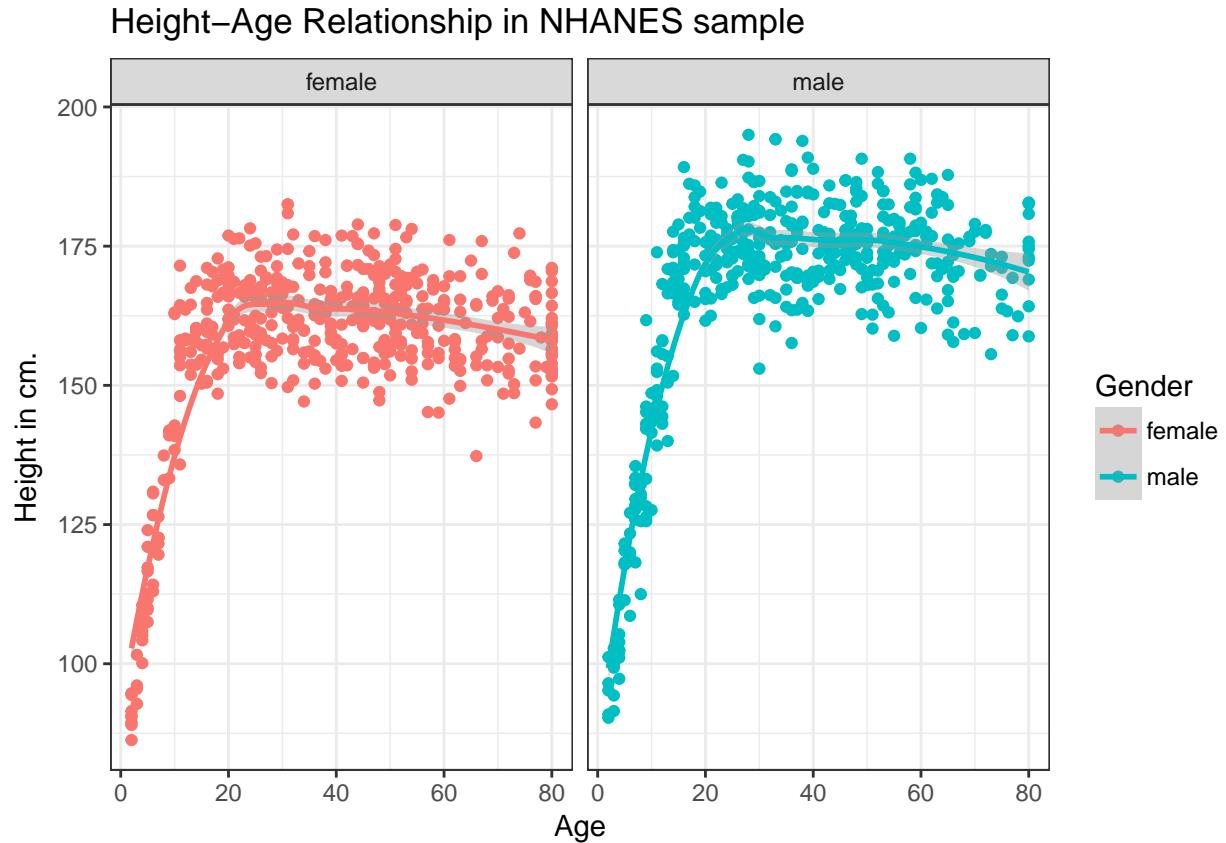
## Height–Age Relationship in NHANES sample



### 3.4.2 Can we add a smooth curve to show the relationship in each plot?

Yep, and let's change the theme of the graph to remove the gray background, too.

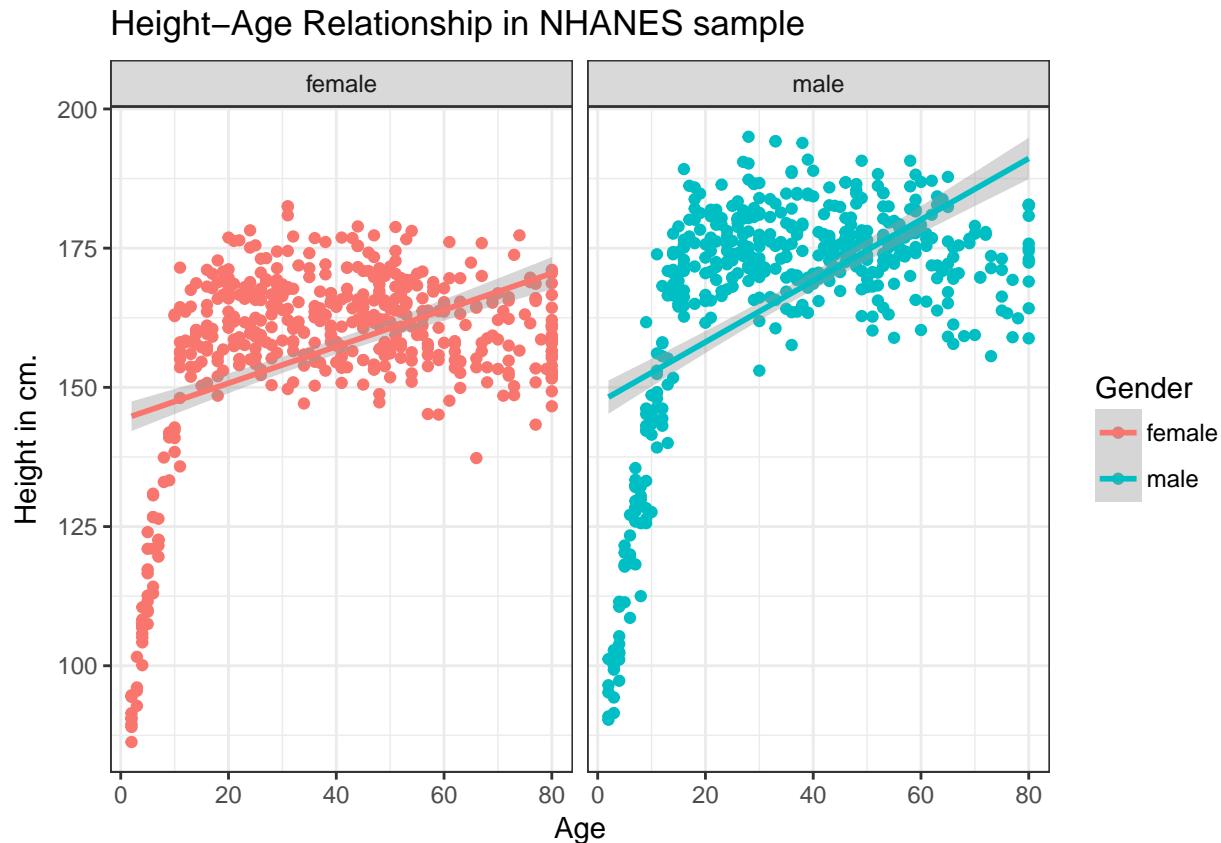
```
ggplot(data = nh_dat2, aes(x = Age, y = Height, color = Gender)) +  
  geom_point() +  
  geom_smooth(method = "loess") +  
  labs(title = "Height-Age Relationship in NHANES sample",  
       y = "Height in cm.") +  
  theme_bw() +  
  facet_wrap(~ Gender)
```



### 3.4.3 What if we want to assume straight line relationships?

We could look at a linear model in the plot. Does this make sense here?

```
ggplot(data = nh_dat2, aes(x = Age, y = Height, color = Gender)) +
  geom_point() +
  geom_smooth(method = "lm") +
  labs(title = "Height–Age Relationship in NHANES sample",
       y = "Height in cm.") +
  theme_bw() +
  facet_wrap(~ Gender)
```



### 3.5 A Subset: Ages 21-79

Suppose we wanted to look at a subset of our sample - those observations (subjects) whose Age is at least 21 and at most 79. We'll create that sample below, and also subset the variables to include nine of particular interest, and remove any observations with any missingness on *any* of the nine variables we're including here.

```
nh_data_2179 <- nh_data %>%
  filter(Age > 20 & Age < 80) %>%
  select(ID, Gender, Age, Height, Weight, BMI, Pulse, Race1, HealthGen, Diabetes) %>%
  na.omit
```

```
nh_data_2179
```

```
# A tibble: 594 x 10
  ID   Gender   Age  Height  Weight    BMI  Pulse  Race1 HealthGen
  <int> <fctr> <int>  <dbl>   <dbl>  <dbl> <int> <fctr>   <fctr>
1 59640   male    54    176  129.0  41.8    74  White    Good
2 59826 female   67    156  50.2   20.5    66  White   Vgood
3 56747   male    33    194 105.1   27.9    68  White   Vgood
4 63908   male    55    169  90.6   31.9    62 Mexican Vgood
5 60865 female   25    156  55.0   22.8    58  Other   Vgood
6 66642   male    41    178  89.3   28.2    72  White   Vgood
7 59880 female   45    163  98.3   36.9    80 Hispanic Good
8 71784 female   24    161  50.2   19.3    72  White   Vgood
9 67616   male    63    184  70.0   20.6    82  White   Vgood
```

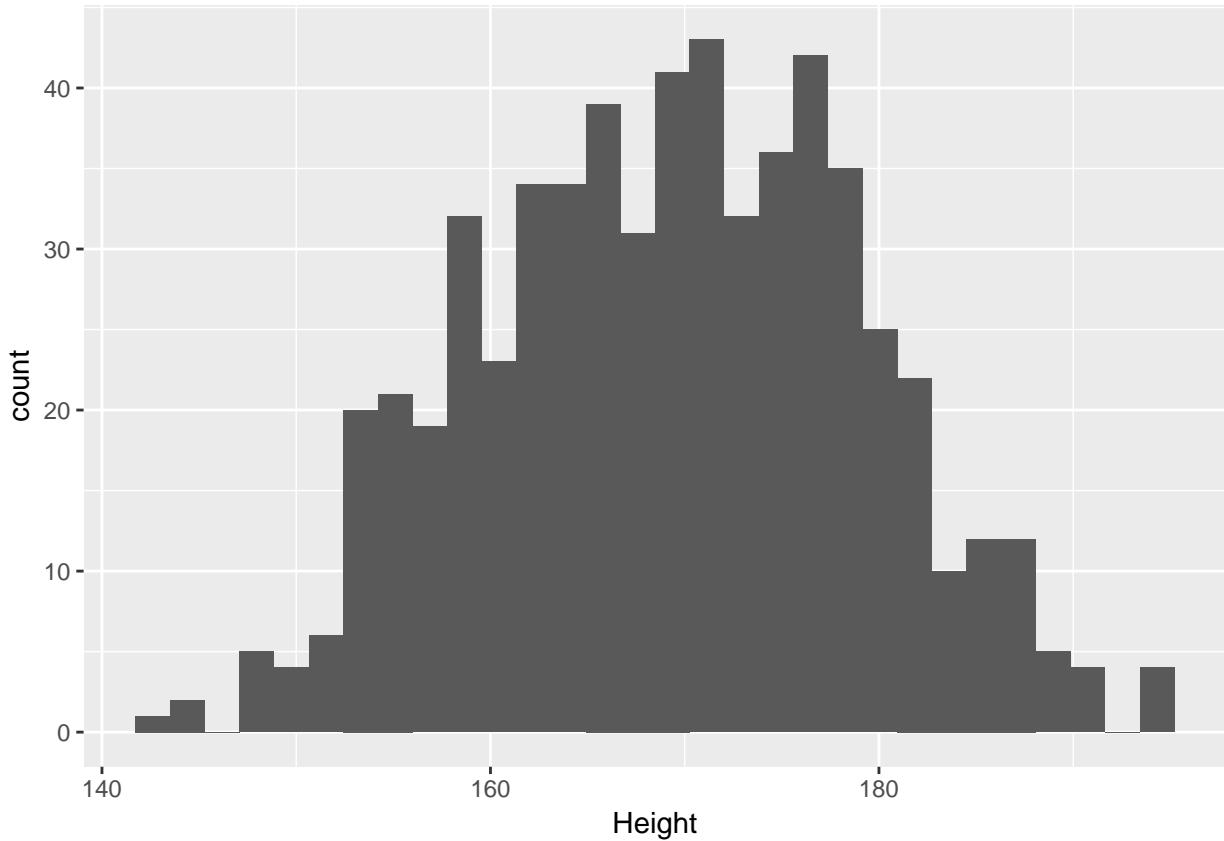
```
10 55391 female    32     161    69.2   26.6   114    Other      Good
# ... with 584 more rows, and 1 more variables: Diabetes <fctr>
```

## 3.6 Distribution of Heights

What is the distribution of height in this new sample?

```
ggplot(data = nh_data_2179, aes(x = Height)) +
  geom_histogram()

`stat_bin()` using `bins = 30`. Pick better value with `binwidth`.
```

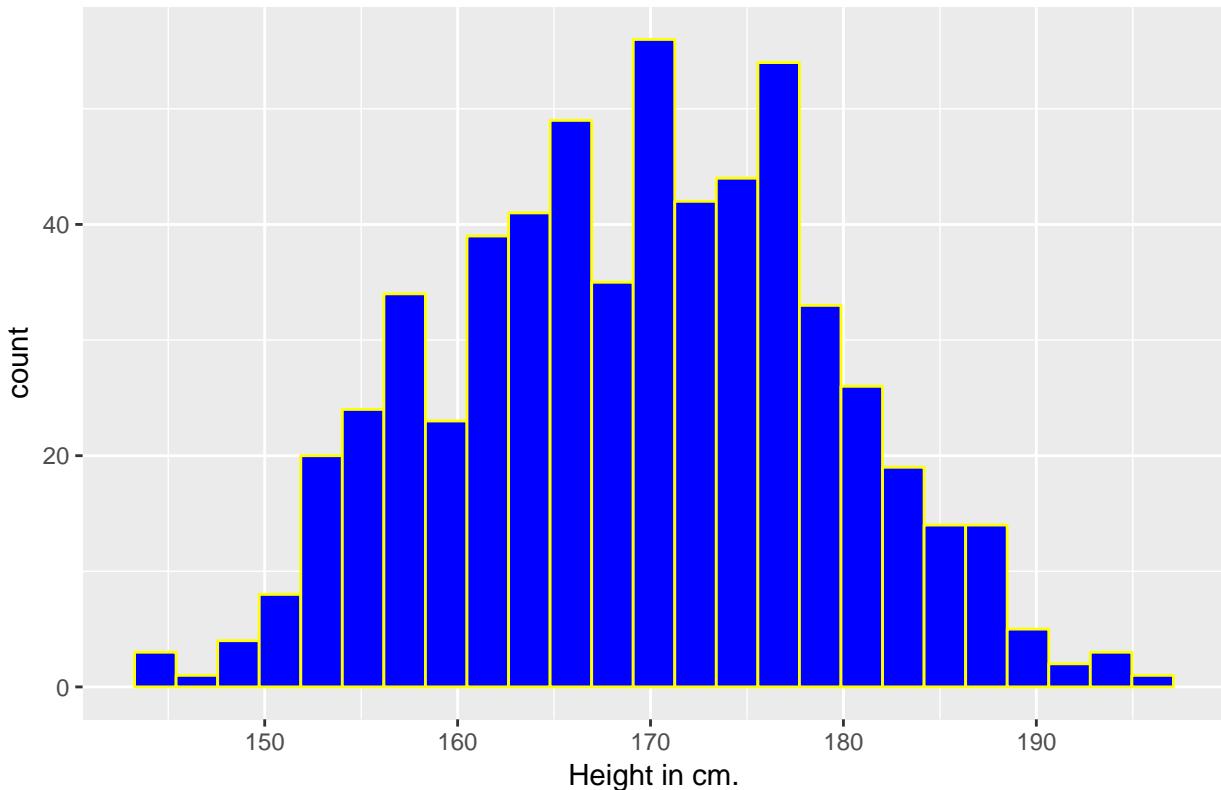


We can do several things to clean this up.

1. We'll change the color of the lines for each bar of the histogram.
2. We'll change the fill inside each bar to make them stand out a bit more.
3. We'll add a title and relabel the horizontal (x) axis to include the units of measurement.
4. We'll avoid the warning by selecting a number of bins (we'll use 25 here) into which we'll group the heights before drawing the histogram.

```
ggplot(data = nh_data_2179, aes(x = Height)) +
  geom_histogram(bins = 25, col = "yellow", fill = "blue") +
  labs(title = "Height of NHANES subjects ages 21-79",
       x = "Height in cm.")
```

### Height of NHANES subjects ages 21–79



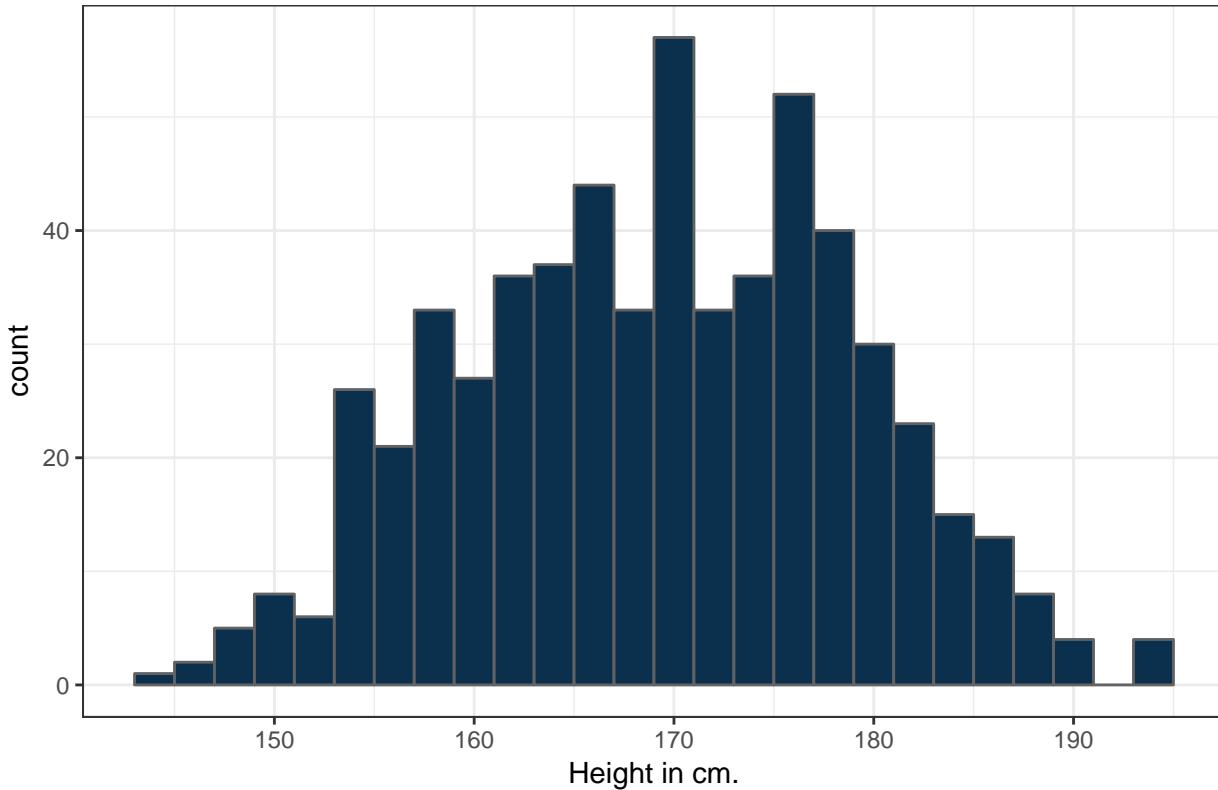
#### 3.6.1 Changing a Histogram's Fill and Color

The CWRU color guide (<https://case.edu/umc/our-brand/visual-guidelines/>) lists the HTML color schemes for CWRU blue and CWRU gray. Let's match that color scheme.

```
cwru.blue <- '#0a304e'
cwru.gray <- '#626262'

ggplot(data = nh_data_2179, aes(x = Height)) +
  geom_histogram(binwidth = 2, col = cwru.gray, fill = cwru.blue) +
  labs(title = "Height of NHANES subjects ages 21-79",
       x = "Height in cm.") +
  theme_bw()
```

### Height of NHANES subjects ages 21–79

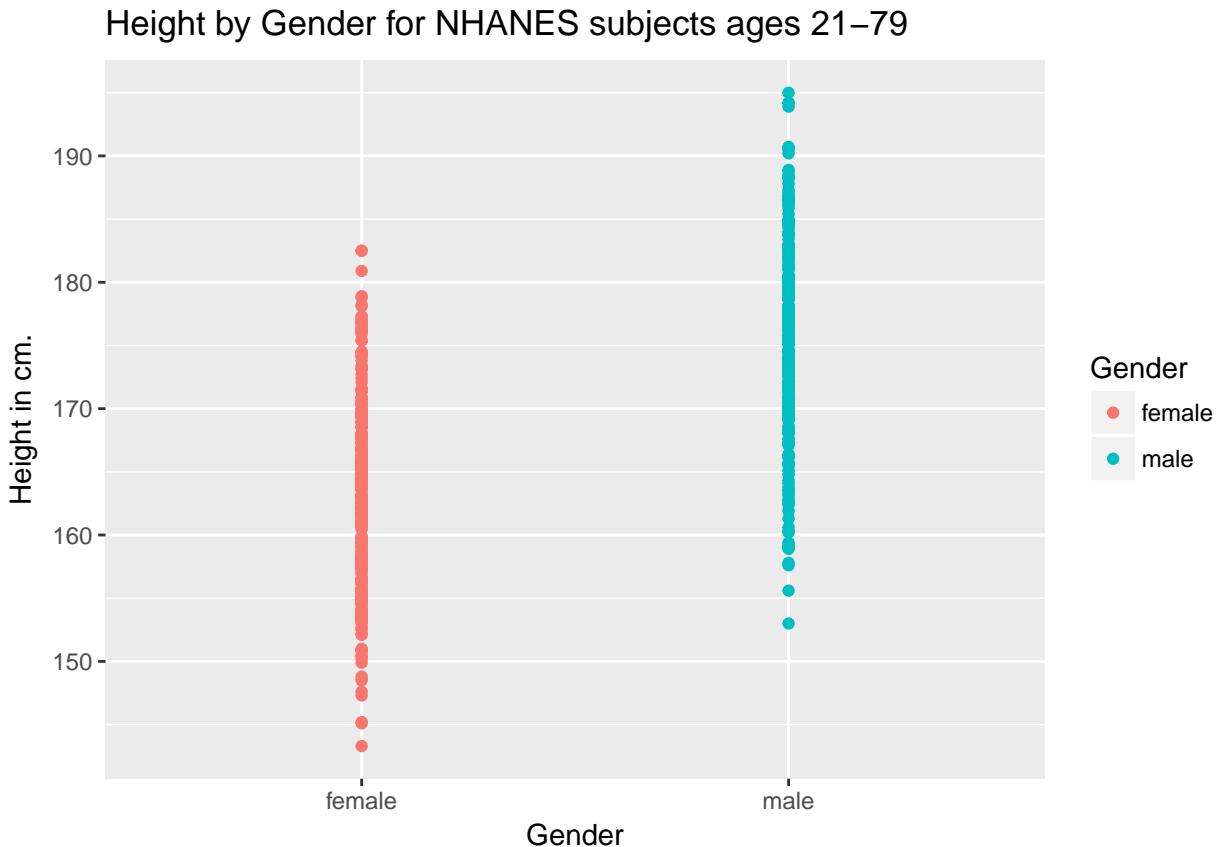


Note the other changes to the graph above.

1. We changed the theme to replace the gray background.
2. We changed the bins for the histogram, to gather observations into groups of 2 cm. each.

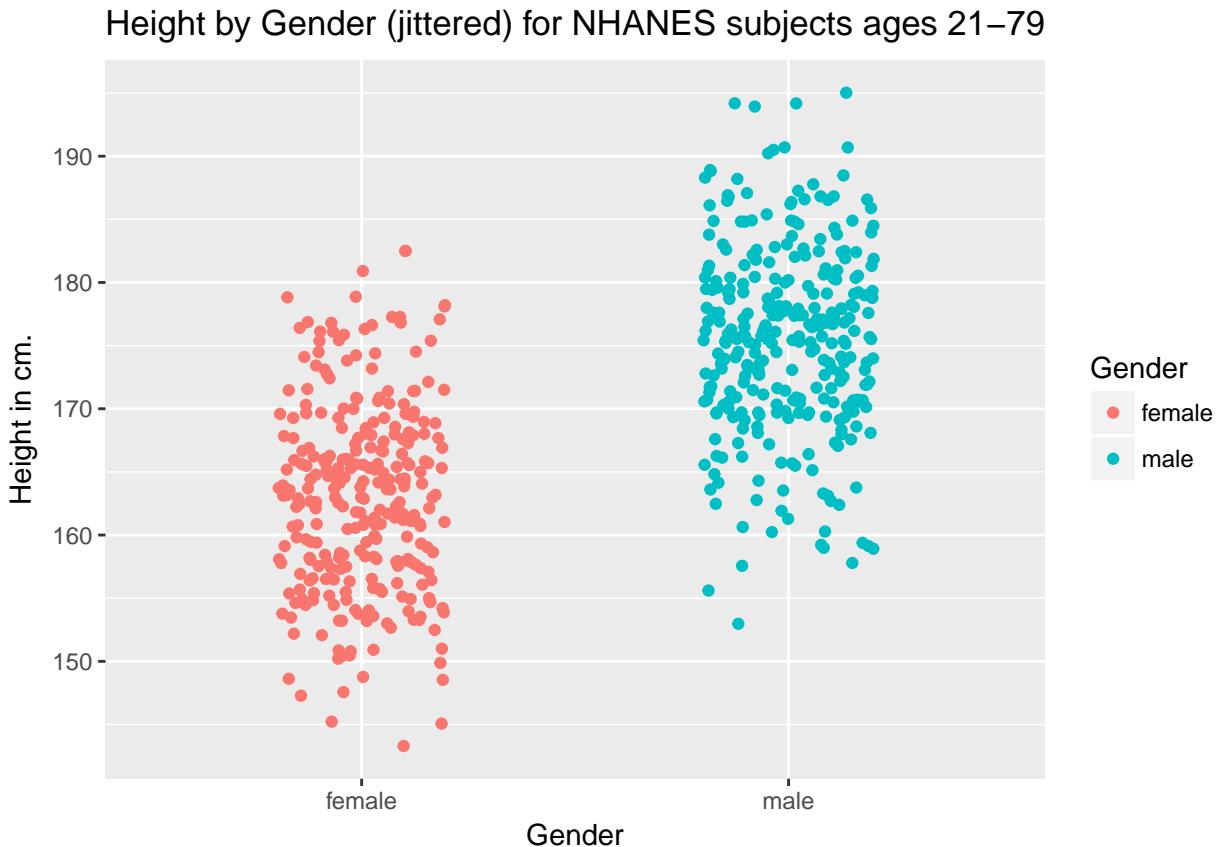
## 3.7 Height and Gender

```
ggplot(data = nh_data_2179, aes(x = Gender, y = Height, color = Gender)) +
  geom_point() +
  labs(title = "Height by Gender for NHANES subjects ages 21-79",
       y = "Height in cm.")
```



This plot isn't so useful. We can improve things a little by jittering the points horizontally, so that the overlap is reduced.

```
ggplot(data = nh_data_2179, aes(x = Gender, y = Height, color = Gender)) +
  geom_jitter(width = 0.2) +
  labs(title = "Height by Gender (jittered) for NHANES subjects ages 21-79",
       y = "Height in cm.")
```

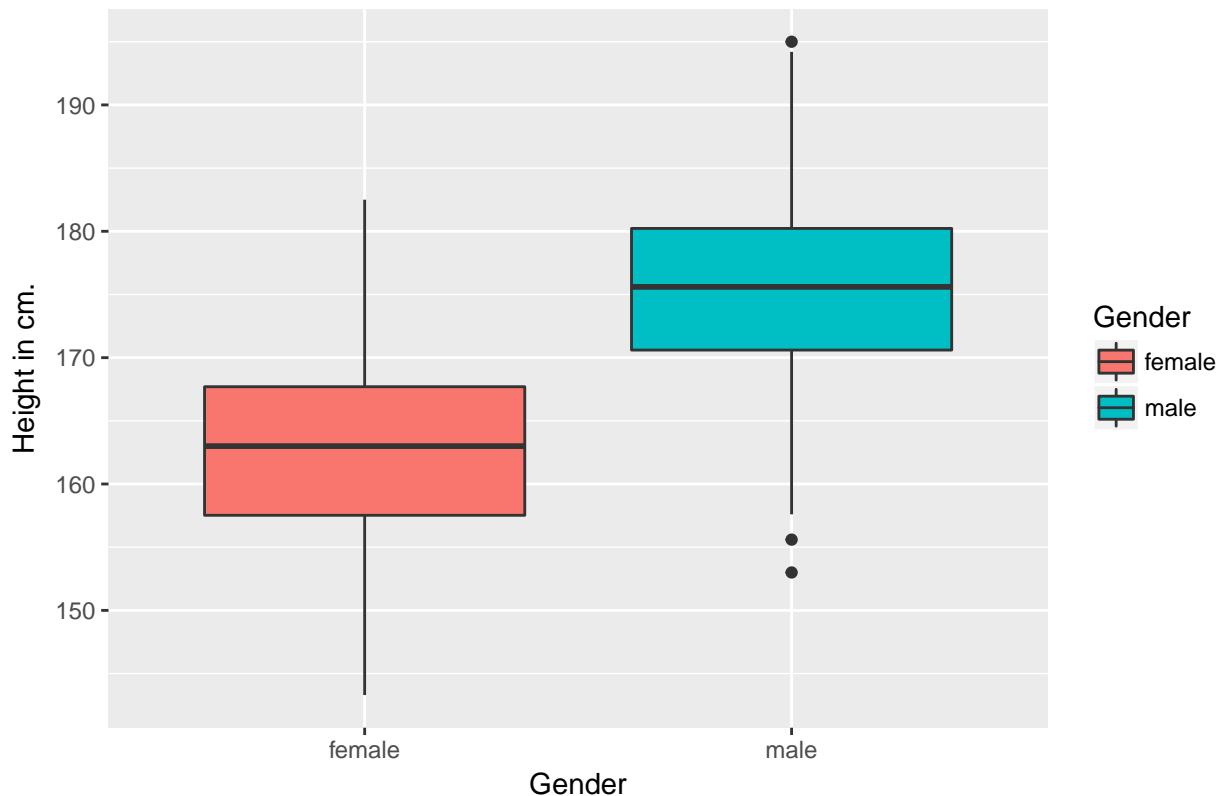


Perhaps it might be better to summarise the distribution in a different way. We might consider a boxplot of the data.

### 3.7.1 A Boxplot of Height by Gender

```
ggplot(data = nh_data_2179, aes(x = Gender, y = Height, fill = Gender)) +
  geom_boxplot() +
  labs(title = "Boxplot of Height by Gender for NHANES subjects ages 21-79",
       y = "Height in cm.")
```

Boxplot of Height by Gender for NHANES subjects ages 21–79

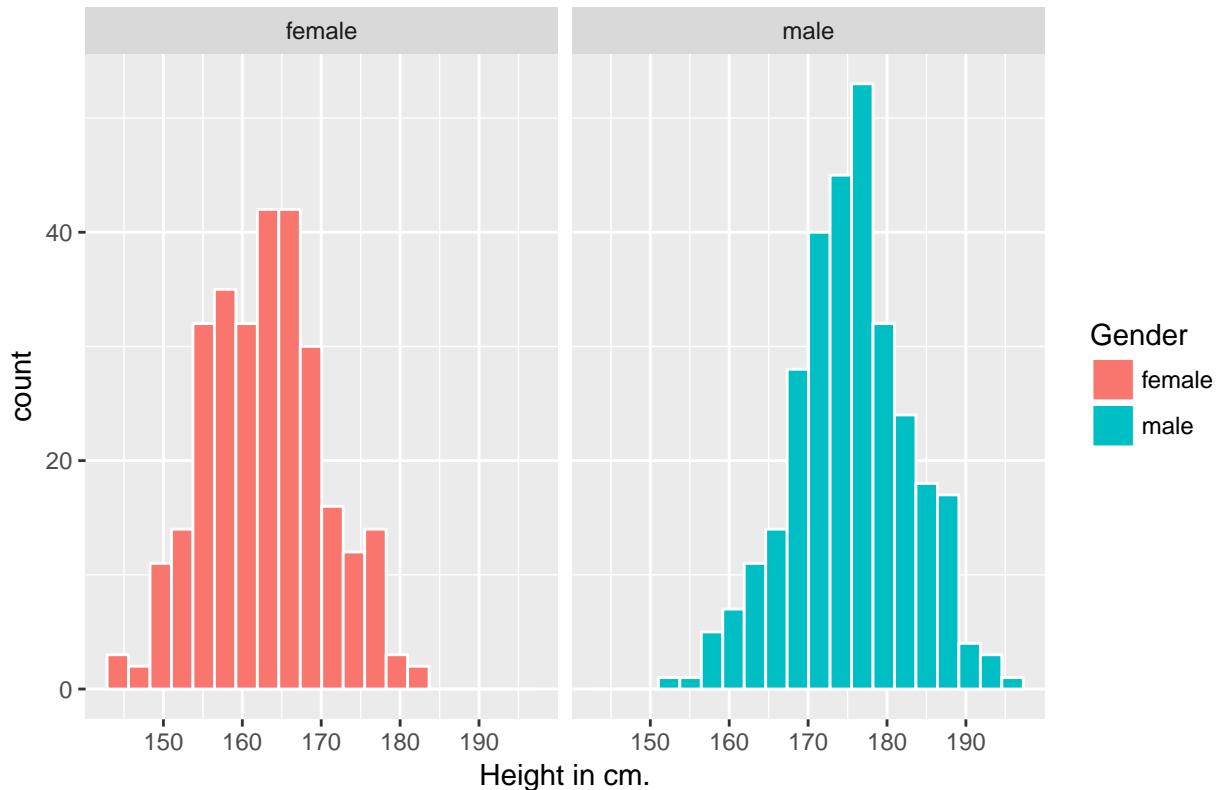


Or perhaps we'd like to see a pair of histograms?

### 3.7.2 Histograms of Height by Gender

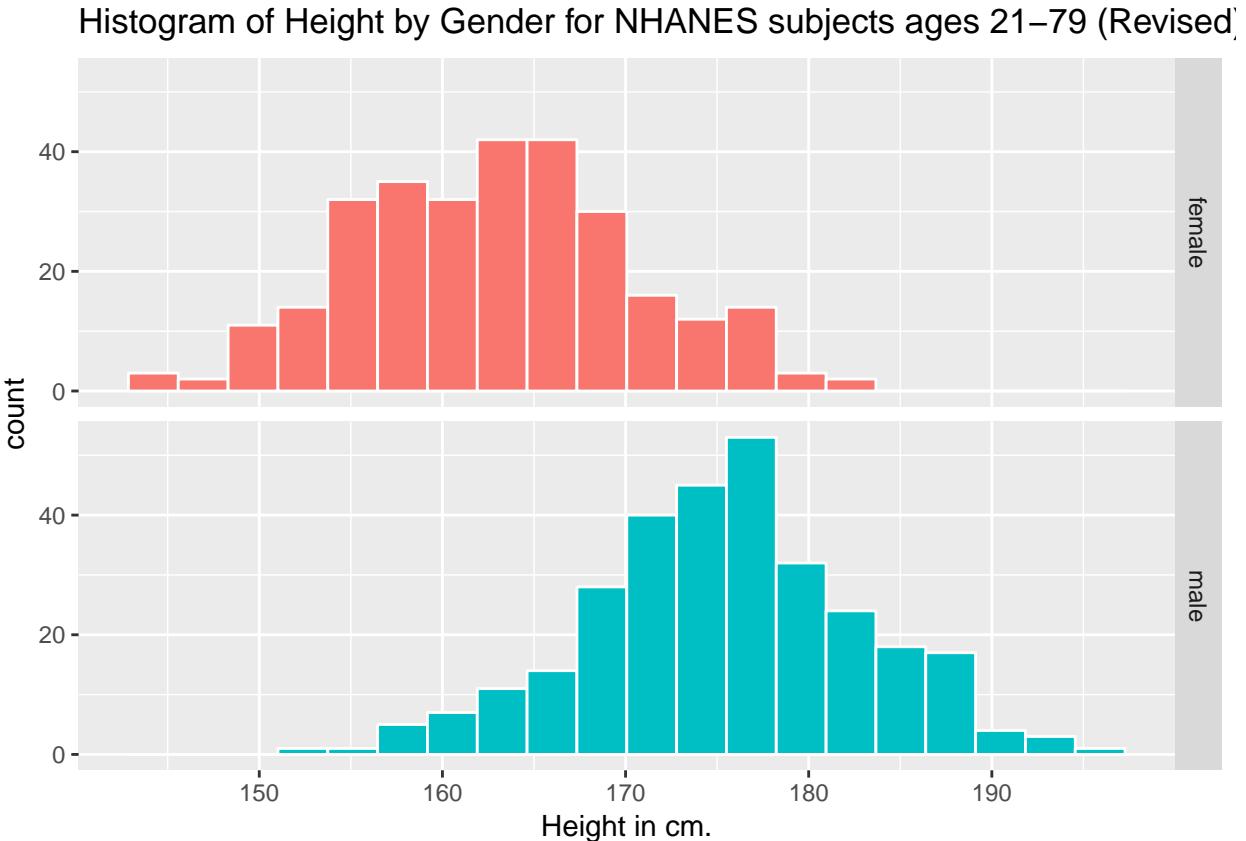
```
ggplot(data = nh_data_2179, aes(x = Height, fill = Gender)) +  
  geom_histogram(color = "white", bins = 20) +  
  labs(title = "Histogram of Height by Gender for NHANES subjects ages 21-79",  
       x = "Height in cm.") +  
  facet_wrap(~ Gender)
```

### Histogram of Height by Gender for NHANES subjects ages 21–79



Can we redraw these histograms so that they are a little more comparable, and to get rid of the unnecessary legend?

```
ggplot(data = nh_data_2179, aes(x = Height, fill = Gender)) +
  geom_histogram(color = "white", bins = 20) +
  labs(title = "Histogram of Height by Gender for NHANES subjects ages 21-79 (Revised)",
       x = "Height in cm.") +
  guides(fill = FALSE) +
  facet_grid(Gender ~ .)
```



### 3.8 A Look at Body-Mass Index

Let's look at a different outcome, the *body-mass index*, or BMI. The definition of BMI for adult subjects (which is expressed in units of  $\text{kg}/\text{m}^2$ ) is:

$$\text{Body Mass Index} = \frac{\text{weight in kg}}{(\text{height in meters})^2} = 703 \times \frac{\text{weight in pounds}}{(\text{height in inches})^2}$$

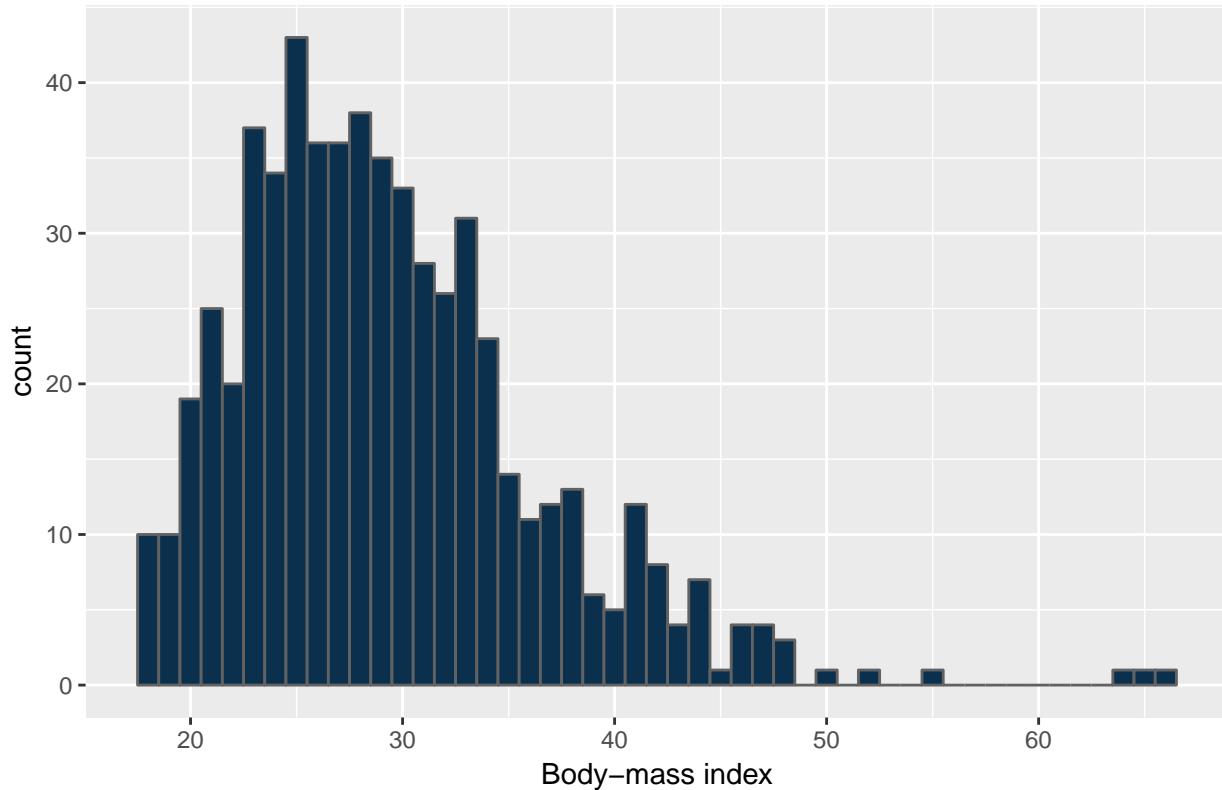
[BMI is essentially] ... a measure of a person's *thinness* or *thickness*... BMI was designed for use as a simple means of classifying average sedentary (physically inactive) populations, with an average body composition. For these individuals, the current value recommendations are as follow: a BMI from 18.5 up to 25 may indicate optimal weight, a BMI lower than 18.5 suggests the person is underweight, a number from 25 up to 30 may indicate the person is overweight, and a number from 30 upwards suggests the person is obese.

Wikipedia, [https://en.wikipedia.org/wiki/Body\\_mass\\_index](https://en.wikipedia.org/wiki/Body_mass_index)

Here's a histogram, again with CWRU colors, for the BMI data.

```
ggplot(data = nh_data_2179, aes(x = BMI)) +
  geom_histogram(binwidth = 1, fill = cwrugrey, col = cwrugray) +
  labs(title = "Histogram of BMI: NHANES subjects ages 21-79",
       x = "Body-mass index")
```

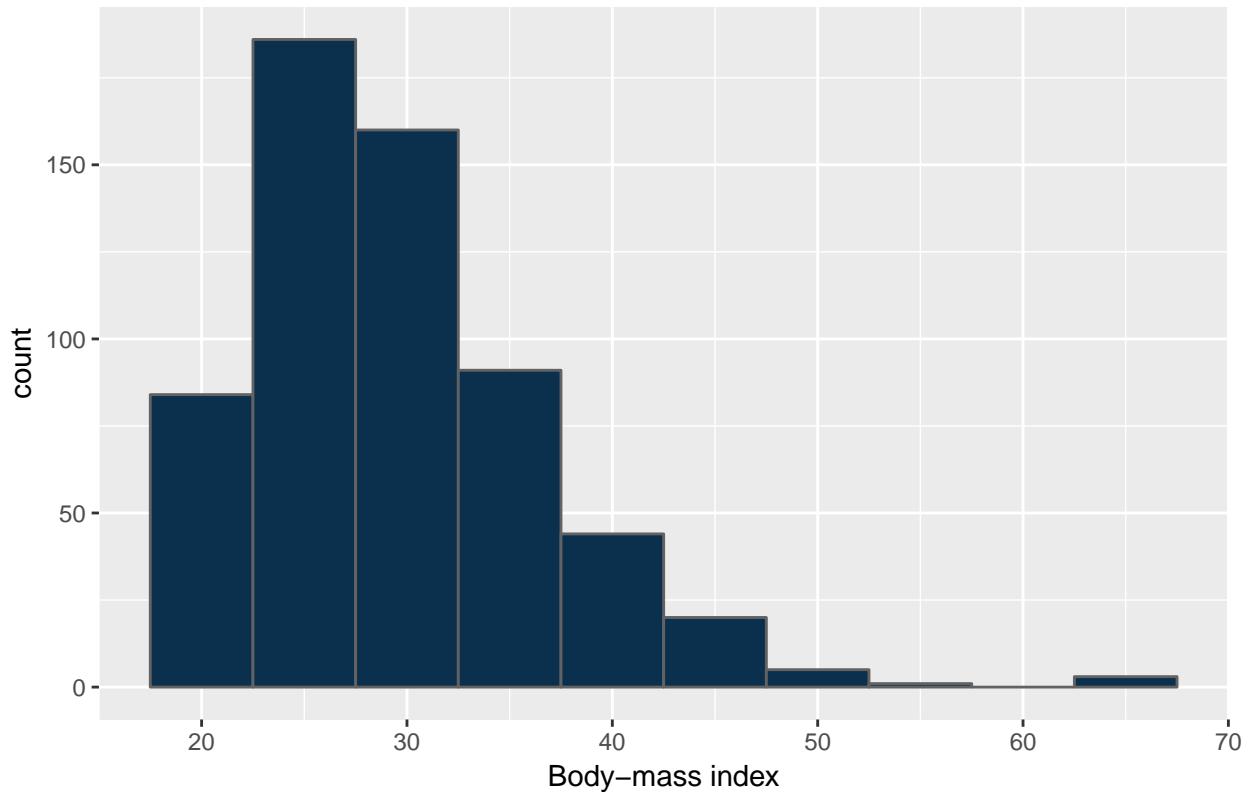
### Histogram of BMI: NHANES subjects ages 21–79



Note how different this picture looks if instead we bin up groups of  $5 \text{ kg/m}^2$  at a time. Which is the more useful representation will depend a lot on what questions you're trying to answer.

```
ggplot(data = nh_data_2179, aes(x = BMI)) +
  geom_histogram(binwidth = 5, fill = cwrugrey, col = cwrugrey) +
  labs(title = "Histogram of BMI: NHANES subjects ages 21–79",
       x = "Body-mass index")
```

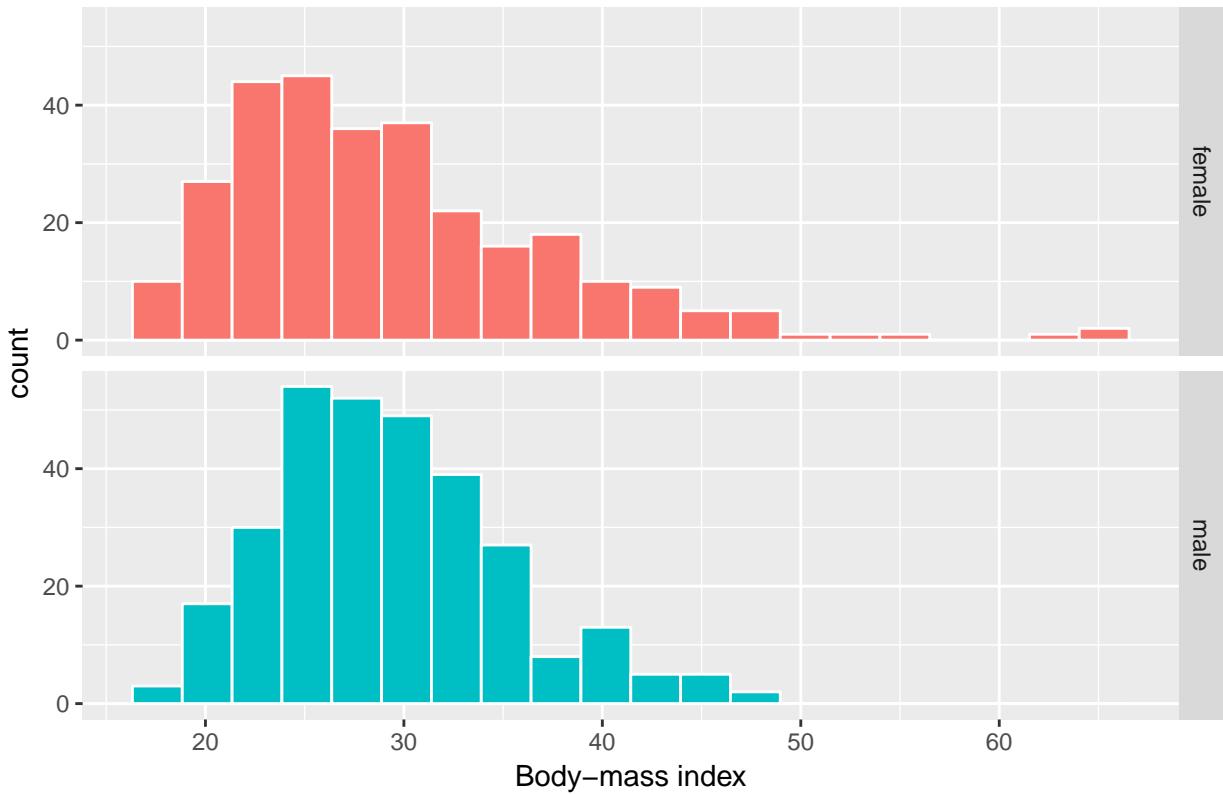
Histogram of BMI: NHANES subjects ages 21–79



### 3.8.1 BMI by Gender

```
ggplot(data = nh_data_2179, aes(x = BMI, fill = Gender)) +
  geom_histogram(color = "white", bins = 20) +
  labs(title = "Histogram of BMI by Gender for NHANES subjects ages 21-79",
       x = "Body-mass index") +
  guides(fill = FALSE) +
  facet_grid(Gender ~ .)
```

### Histogram of BMI by Gender for NHANES subjects ages 21–79



As an accompanying numerical summary, we might ask how many people fall into each of these Gender categories, and what is their “average” BMI.

```
nh_data_2179 %>%
  group_by(Gender) %>%
  summarise(count = n(), mean(BMI), median(BMI)) %>%
  knitr::kable()
```

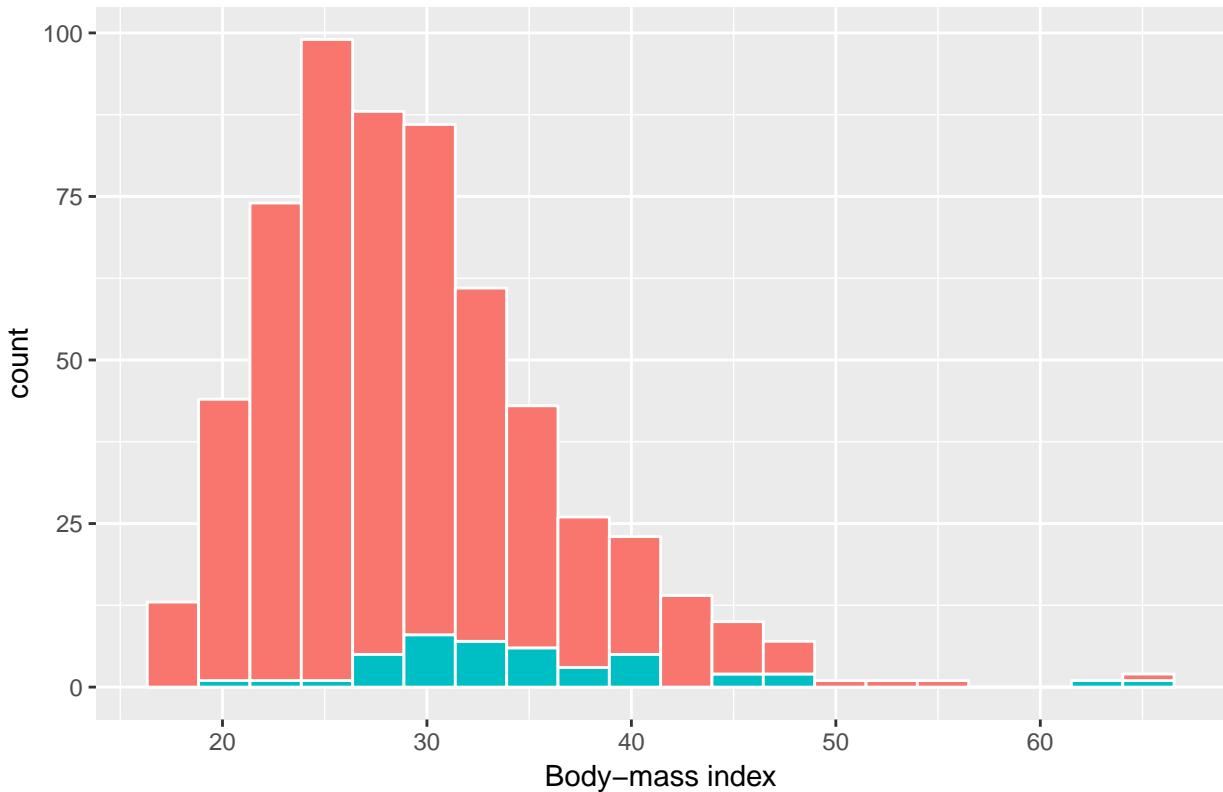
Gender	count	mean(BMI)	median(BMI)
female	290	29.4	27.4
male	304	29.4	28.7

### 3.8.2 BMI and Diabetes

We can split up our histogram into groups based on whether the subjects have been told they have diabetes.

```
ggplot(data = nh_data_2179, aes(x = BMI, fill = Diabetes)) +
  geom_histogram(color = "white", bins = 20) +
  labs(title = "BMI by Diabetes Status for NHANES ages 21-79",
       x = "Body-mass index") +
  guides(fill = FALSE)
```

### BMI by Diabetes Status for NHANES ages 21–79



How many people fall into each of these Diabetes categories, and what is their “average” BMI?

```
nh_data_2179 %>%
  group_by(Diabetes) %>%
  summarise(count = n(), mean(BMI), median(BMI)) %>%
  knitr::kable()
```

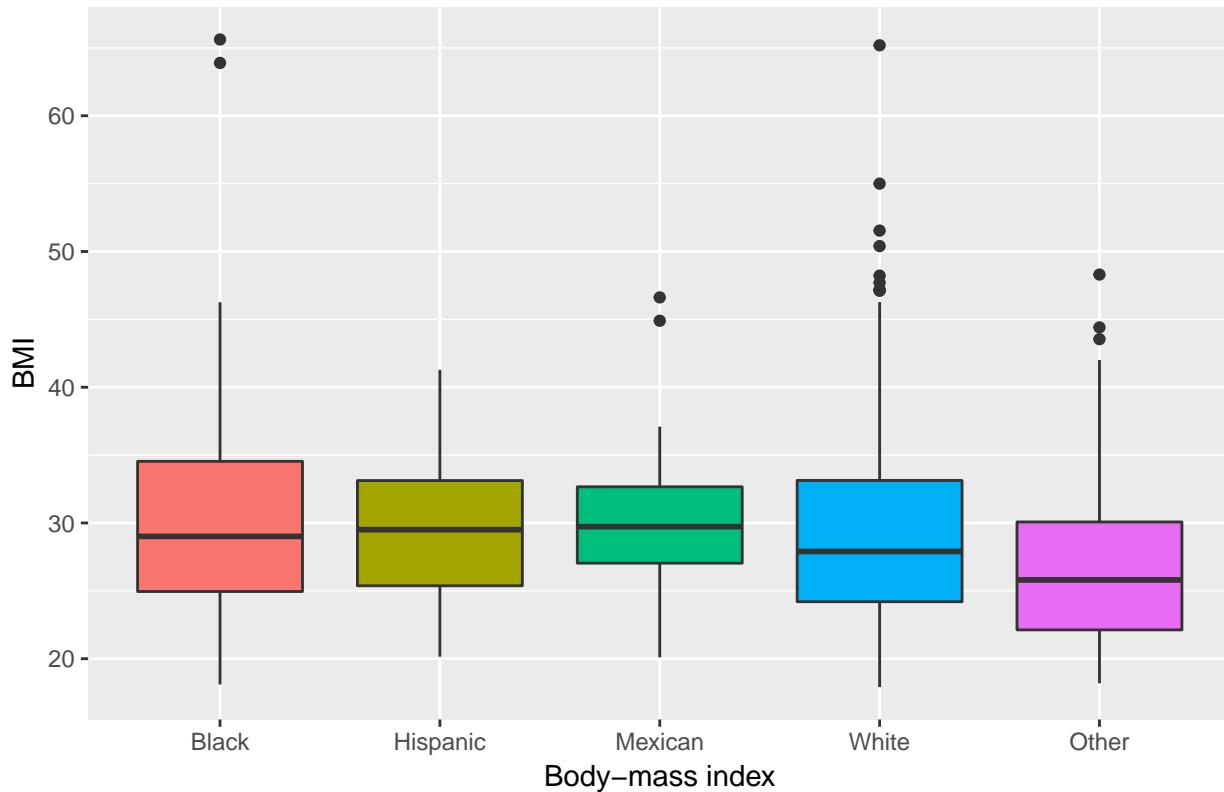
Diabetes	count	mean(BMI)	median(BMI)
No	551	28.9	27.9
Yes	43	35.3	33.4

#### 3.8.3 BMI and Race

We can compare the distribution of BMI across Race groups, as well.

```
ggplot(data = nh_data_2179, aes(x = Race1, y = BMI, fill = Race1)) +
  geom_boxplot() +
  labs(title = "BMI by Race for NHANES ages 21–79",
       x = "Body-mass index") +
  guides(fill = FALSE)
```

### BMI by Race for NHANES ages 21–79



How many people fall into each of these Race1 categories, and what is their “average” BMI?

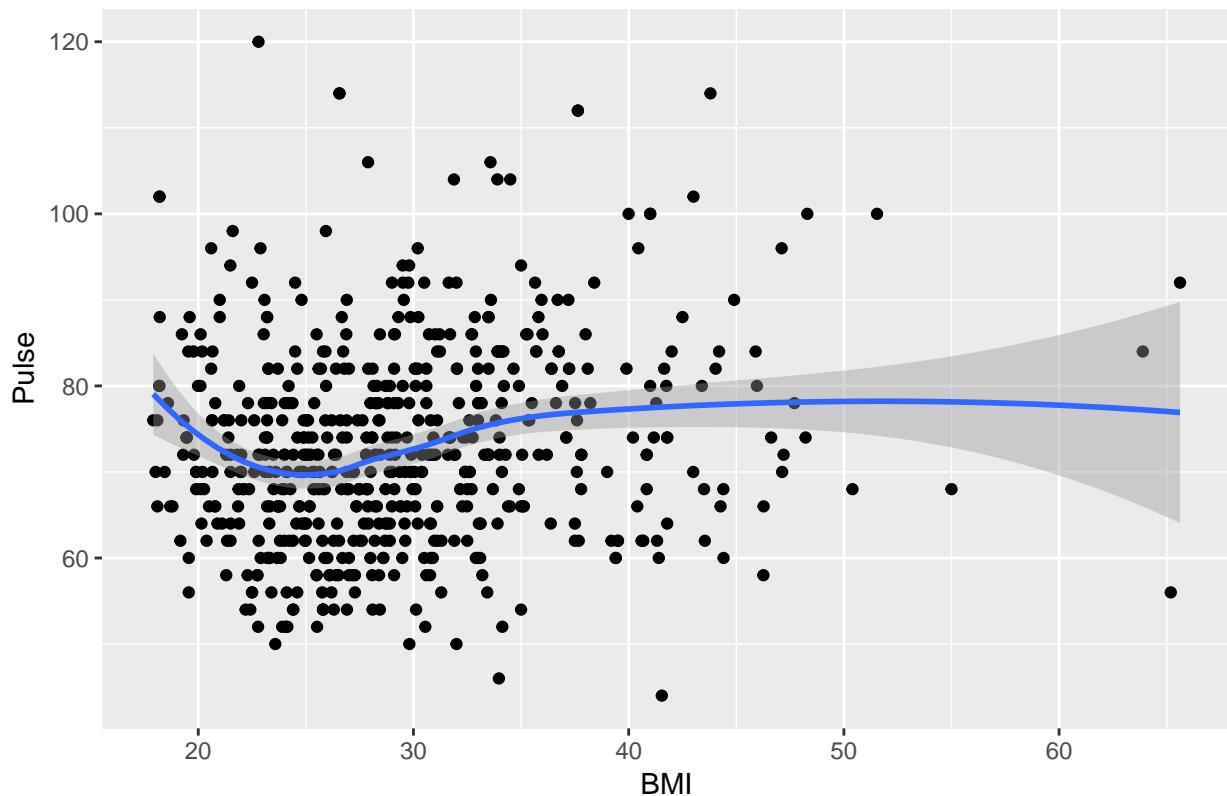
```
library(tidyverse)
nh_data_2179 %>%
  group_by(Race1) %>%
  summarise(count = n(), mean(BMI), median(BMI)) %>%
  knitr::kable()
```

Race1	count	mean(BMI)	median(BMI)
Black	63	31.0	29.0
Hispanic	44	29.4	29.5
Mexican	50	30.0	29.7
White	387	29.3	27.9
Other	50	27.3	25.8

#### 3.8.4 BMI and Pulse Rate

```
ggplot(data = nh_data_2179, aes(x = BMI, y = Pulse)) +
  geom_point() +
  geom_smooth(method = "loess") +
  labs(title = "BMI vs. Pulse rate for NHANES subjects, ages 21-79")
```

### BMI vs. Pulse rate for NHANES subjects, ages 21–79



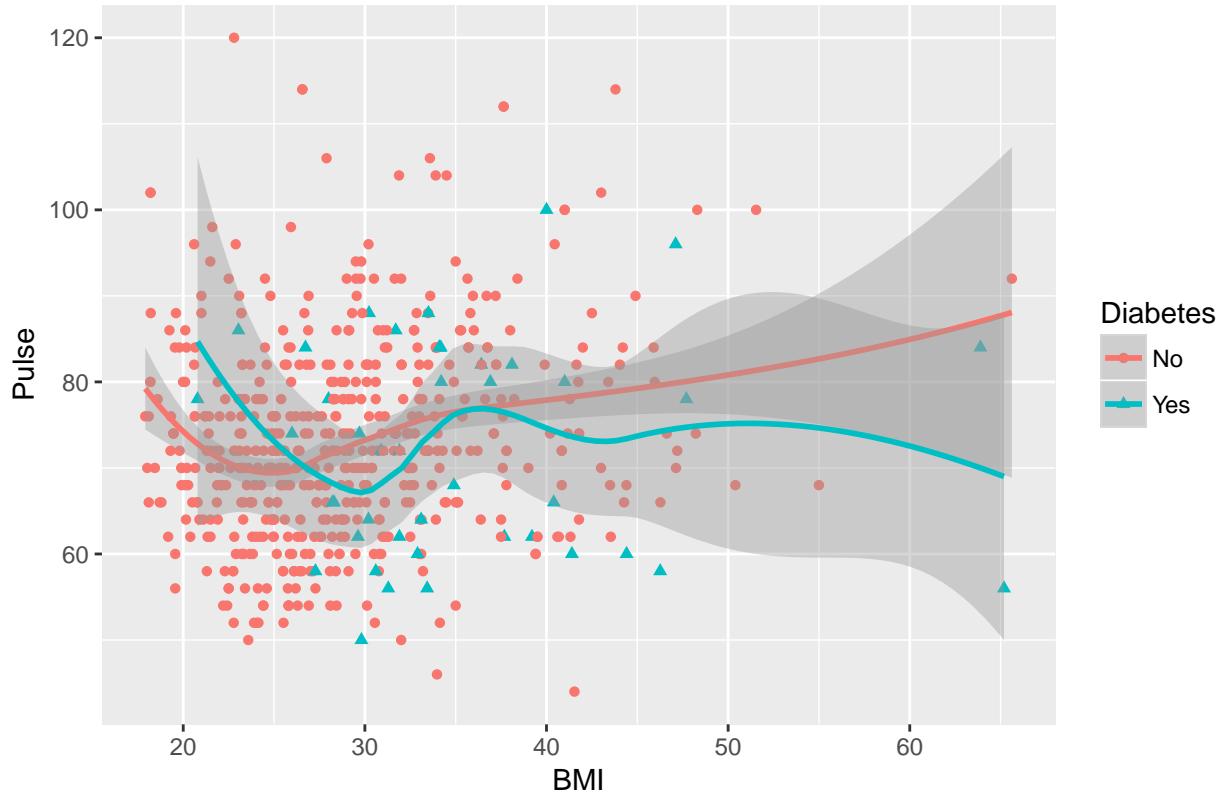
#### 3.8.5 Diabetes vs. No Diabetes

Could we see whether subjects who have been told they have diabetes show different BMI-pulse rate patterns than the subjects who haven't?

- Let's try doing this by changing the shape *and* the color of the points based on diabetes status.

```
ggplot(data = nh_data_2179,
       aes(x = BMI, y = Pulse,
           color = Diabetes, shape = Diabetes)) +
  geom_point() +
  geom_smooth(method = "loess") +
  labs(title = "BMI vs. Pulse rate for NHANES subjects, ages 21-79")
```

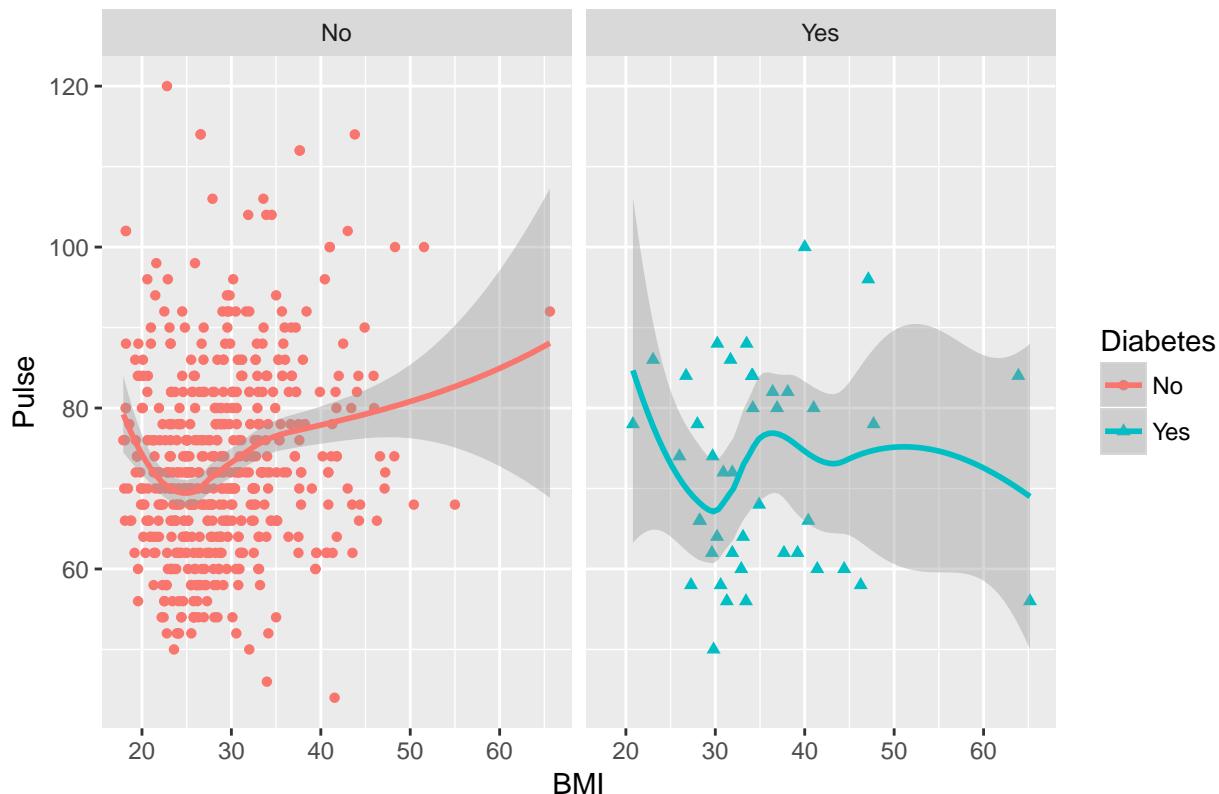
### BMI vs. Pulse rate for NHANES subjects, ages 21–79



This plot might be easier to interpret if we facet by Diabetes status, as well.

```
ggplot(data = nh_data_2179,
       aes(x = BMI, y = Pulse,
            color = Diabetes, shape = Diabetes)) +
  geom_point() +
  geom_smooth(method = "loess") +
  labs(title = "BMI vs. Pulse rate for NHANES subjects, ages 21-79") +
  facet_wrap(~ Diabetes)
```

### BMI vs. Pulse rate for NHANES subjects, ages 21–79



## 3.9 General Health Status

Here's a Table of the General Health Status results. This is a self-reported rating of each subject's health on a five point scale (Excellent, Very Good, Good, Fair, Poor.)

```
nh_data_2179 %>%
  select(HealthGen) %>%
  table()
```

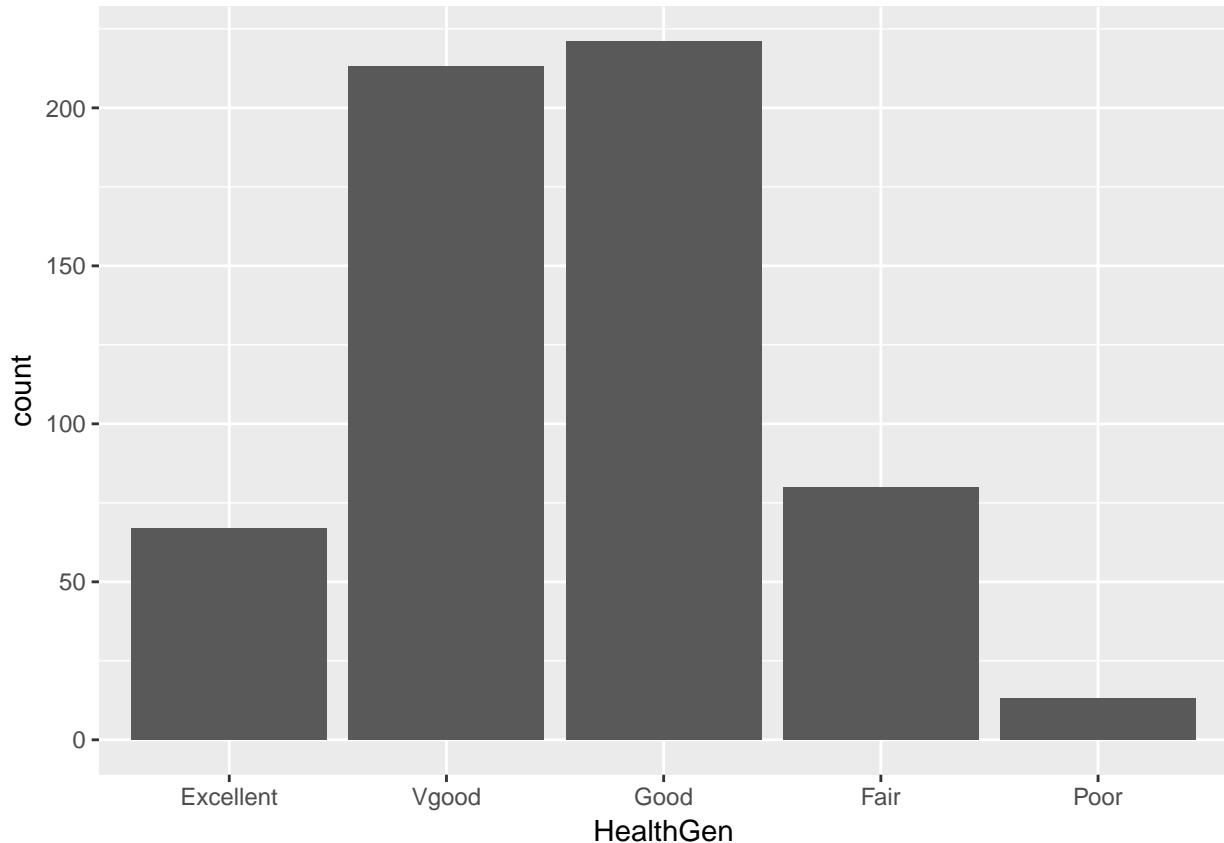
	Excellent	Vgood	Good	Fair	Poor
	67	213	221	80	13

The HealthGen data are categorical, which means that summarizing them with averages isn't as appealing as looking at percentages, proportions and rates.

### 3.9.1 Bar Chart for Categorical Data

Usually, a **bar chart** is the best choice for a graphing a variable made up of categories.

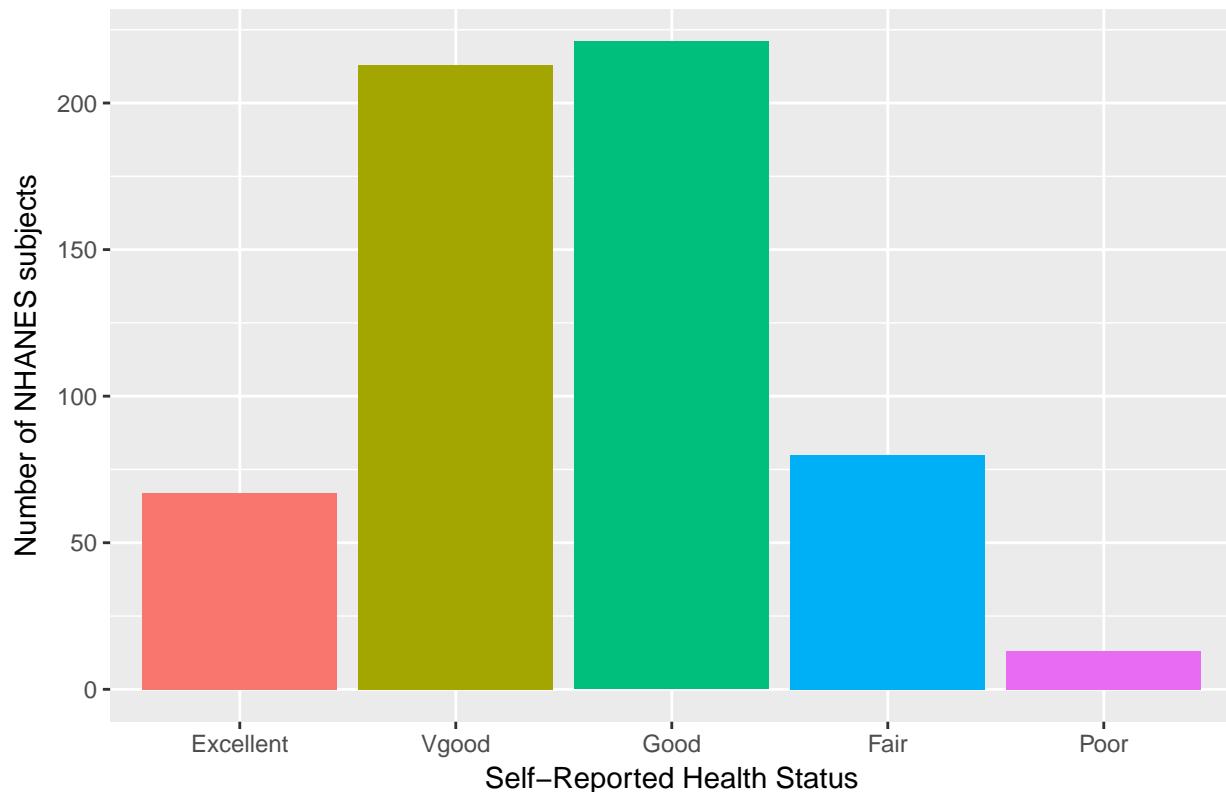
```
ggplot(data = nh_data_2179, aes(x = HealthGen)) +
  geom_bar()
```



There are lots of things we can do to make this plot fancier.

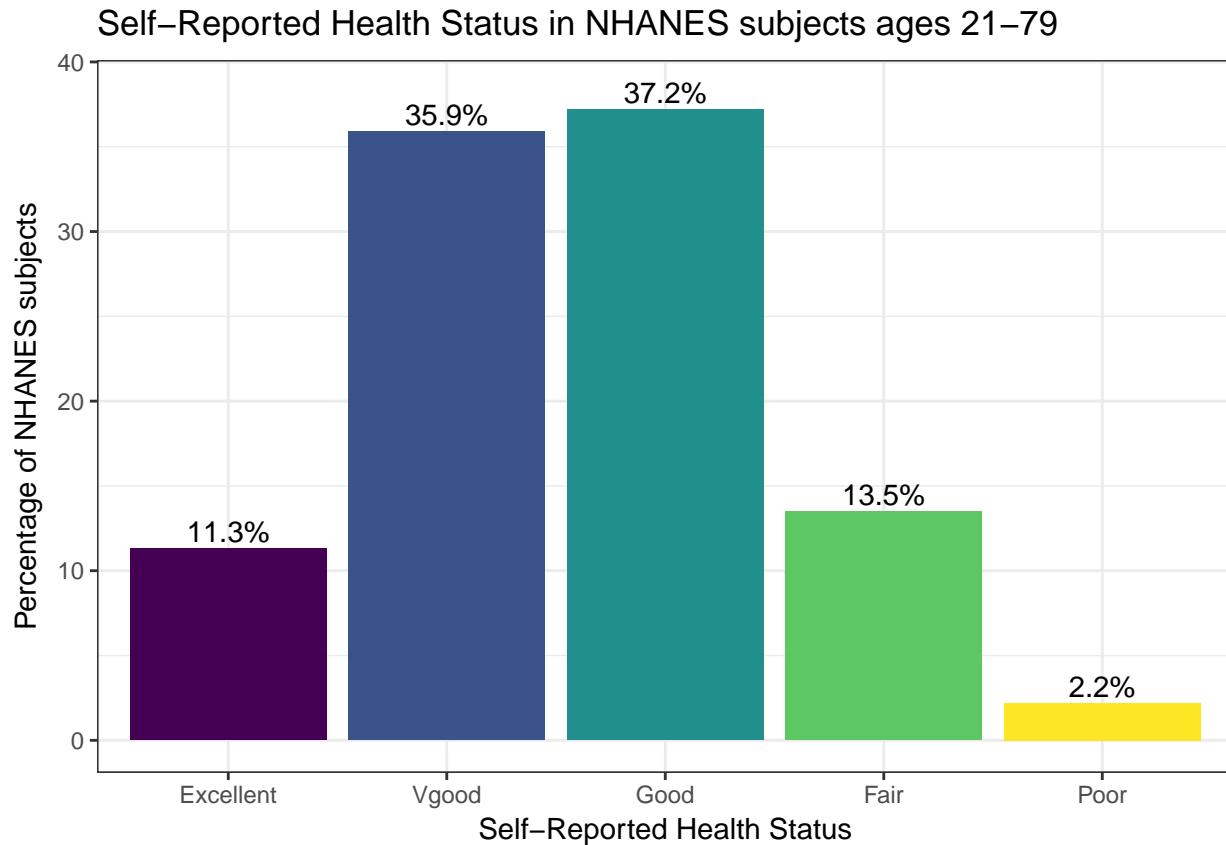
```
ggplot(data = nh_data_2179, aes(x = HealthGen, fill = HealthGen)) +  
  geom_bar() +  
  guides(fill = FALSE) +  
  labs(x = "Self-Reported Health Status",  
       y = "Number of NHANES subjects",  
       title = "Self-Reported Health Status in NHANES subjects ages 21-79")
```

### Self-Reported Health Status in NHANES subjects ages 21–79



Or, we can really go crazy...

```
nh_data_2179 %>%
  count(HealthGen) %>%
  ungroup() %>%
  mutate(pct = round(prop.table(n) * 100, 1)) %>%
  ggplot(aes(x = HealthGen, y = pct, fill = HealthGen)) +
  geom_bar(stat = "identity", position = "dodge") +
  scale_fill_viridis(discrete = TRUE) +
  guides(fill = FALSE) +
  geom_text(aes(y = pct + 1,      # nudge above top of bar
                label = paste0(pct, '%')), # prettify
            position = position_dodge(width = .9),
            size = 4) +
  labs(x = "Self-Reported Health Status",
       y = "Percentage of NHANES subjects",
       title = "Self-Reported Health Status in NHANES subjects ages 21-79") +
  theme_bw()
```



### 3.9.2 Working with Tables

We can add a marginal total, and compare subjects by Gender, as follows...

```
nh_data_2179 %>%
  select(Gender, HealthGen) %>%
  table() %>%
  addmargins()
```

Gender	HealthGen					Sum
	Excellent	Vgood	Good	Fair	Poor	
female	34	107	107	34	8	290
male	33	106	114	46	5	304
Sum	67	213	221	80	13	594

If we like, we can make this look a little more polished with the `knitr::kable` function...

```
nh_data_2179 %>%
  select(Gender, HealthGen) %>%
  table() %>%
  addmargins() %>%
  knitr::kable()
```

	Excellent	Vgood	Good	Fair	Poor	Sum
female	34	107	107	34	8	290
male	33	106	114	46	5	304
Sum	67	213	221	80	13	594

If we want the proportions of patients within each Gender that fall in each HealthGen category (the row percentages), we can get them, too.

```
nh_data_2179 %>%
  select(Gender, HealthGen) %>%
  table() %>%
  prop.table(.,1) %>%
  knitr::kable()
```

	Excellent	Vgood	Good	Fair	Poor
female	0.117	0.369	0.369	0.117	0.028
male	0.109	0.349	0.375	0.151	0.016

To make this a little easier to use, we might consider rounding.

```
nh_data_2179 %>%
  select(Gender, HealthGen) %>%
  table() %>%
  prop.table(.,1) %>%
  round(.,2) %>%
  knitr::kable()
```

	Excellent	Vgood	Good	Fair	Poor
female	0.12	0.37	0.37	0.12	0.03
male	0.11	0.35	0.38	0.15	0.02

Another possibility would be to show the percentages, rather than the proportions (which requires multiplying the proportion by 100.) Note the strange “\*” function, which is needed to convince R to multiply each entry by 100 here.

```
nh_data_2179 %>%
  select(Gender, HealthGen) %>%
  table() %>%
  prop.table(.,1) %>%
  "*"(100) %>%
  round(.,2) %>%
  knitr::kable()
```

	Excellent	Vgood	Good	Fair	Poor
female	11.7	36.9	36.9	11.7	2.76
male	10.9	34.9	37.5	15.1	1.64

And, if we wanted the column percentages, to determine which gender had the higher rate of each HealthGen status level, we can get that by changing the prop.table to calculate 2 (column) proportions, rather than 1 (rows.)

```
nh_data_2179 %>%
  select(Gender, HealthGen) %>%
  table() %>%
  prop.table(.,2) %>%
  "*"(100) %>%
  round(.,2) %>%
  knitr::kable()
```

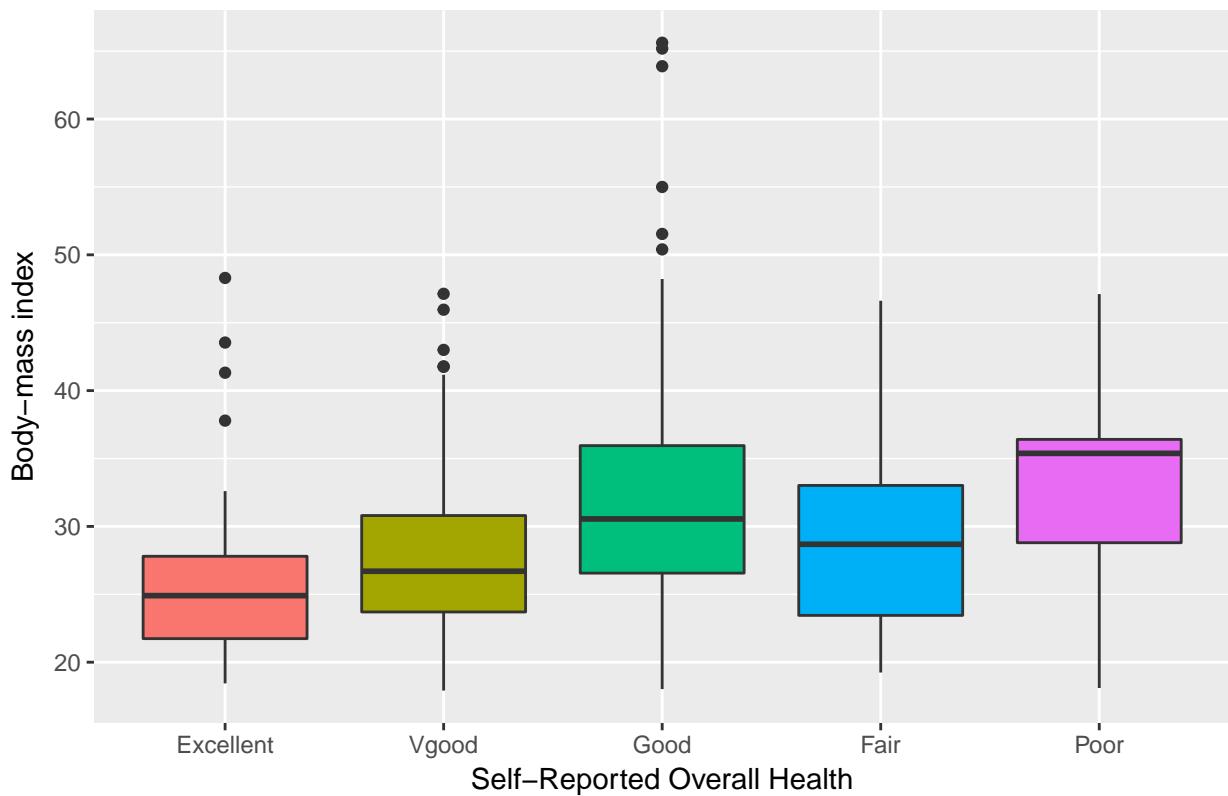
	Excellent	Vgood	Good	Fair	Poor
female	50.8	50.2	48.4	42.5	61.5
male	49.2	49.8	51.6	57.5	38.5

### 3.9.3 BMI by General Health Status

Let's consider now the relationship between self-reported overall health and body-mass index.

```
ggplot(data = nh_data_2179, aes(x = HealthGen, y = BMI, fill = HealthGen)) +
  geom_boxplot() +
  labs(title = "BMI by Health Status, Overall Health for NHANES ages 21-79",
       y = "Body-mass index", x = "Self-Reported Overall Health") +
  guides(fill = FALSE)
```

BMI by Health Status, Overall Health for NHANES ages 21–79



We can see that not too many people self-identify with the “Poor” health category.

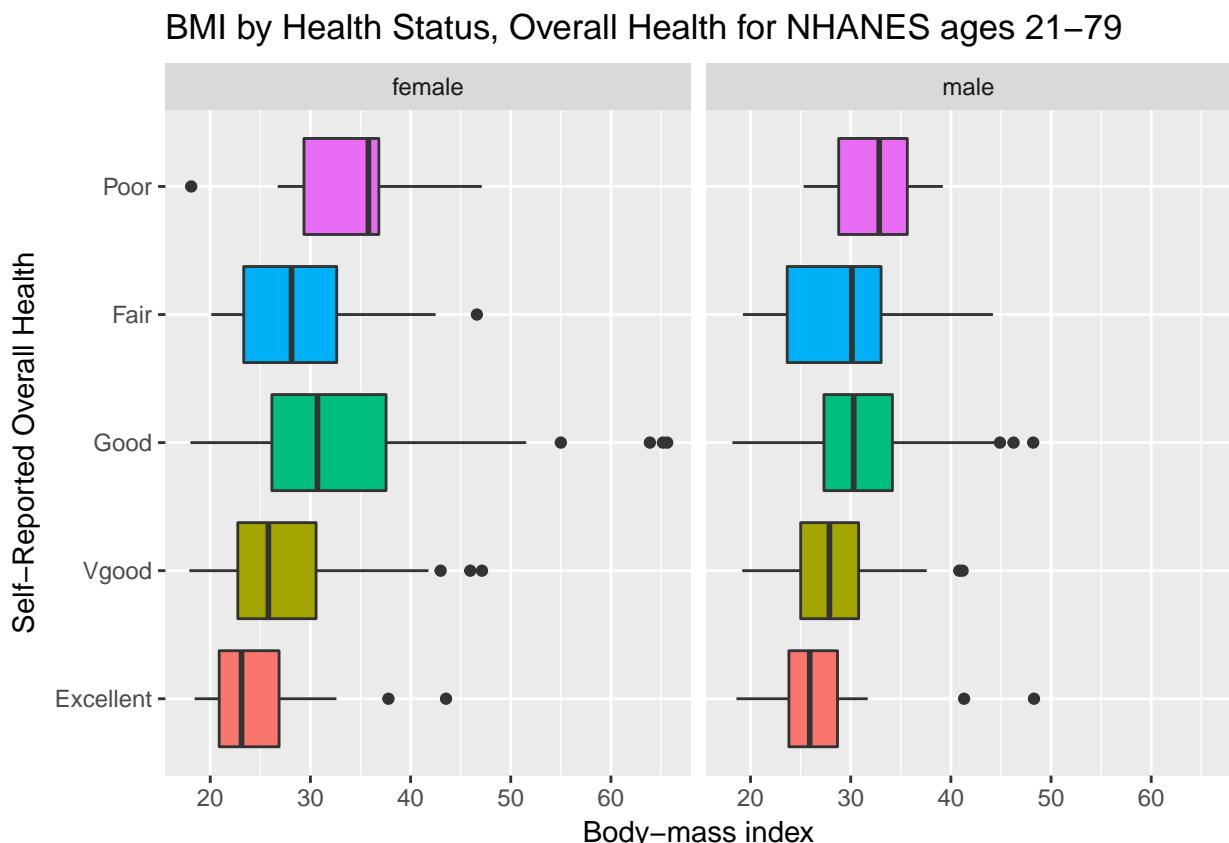
```
nh_data_2179 %>%
  group_by(HealthGen) %>%
  summarise(count = n(), mean(BMI), median(BMI)) %>%
  knitr::kable()
```

HealthGen	count	mean(BMI)	median(BMI)
Excellent	67	25.7	24.9
Vgood	213	27.6	26.7
Good	221	32.0	30.6
Fair	80	29.3	28.7
Poor	13	33.1	35.4

### 3.9.4 BMI by Gender and General Health Status

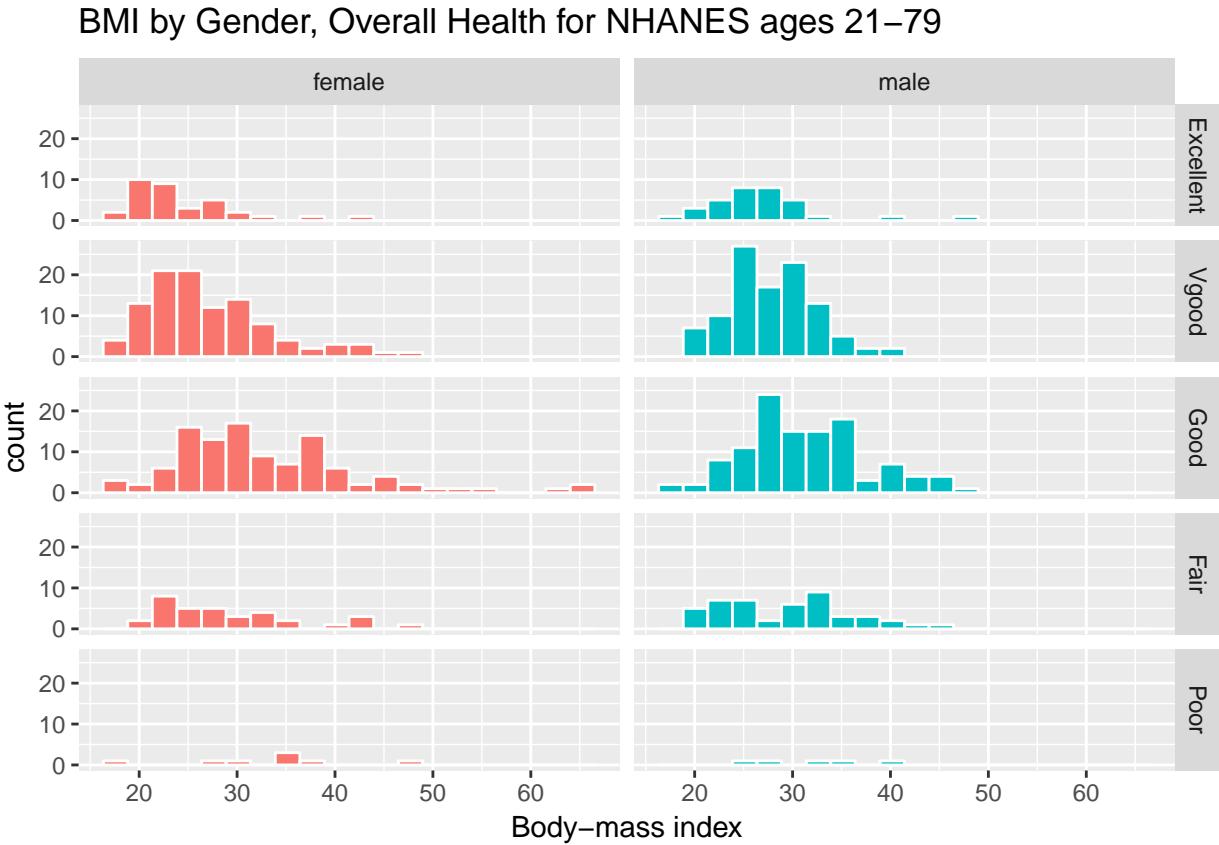
We'll start with two panels of boxplots to try to understand the relationships between BMI, General Health Status and Gender. Note the use of `coord_flip` to rotate the graph 90 degrees.

```
ggplot(data = nh_data_2179, aes(x = HealthGen, y = BMI, fill = HealthGen)) +
  geom_boxplot() +
  labs(title = "BMI by Health Status, Overall Health for NHANES ages 21-79",
       y = "Body-mass index", x = "Self-Reported Overall Health") +
  guides(fill = FALSE) +
  facet_wrap(~ Gender) +
  coord_flip()
```



Here's a plot of faceted histograms, which might be used to address similar questions.

```
ggplot(data = nh_data_2179, aes(x = BMI, fill = Gender)) +
  geom_histogram(color = "white", bins = 20) +
  labs(title = "BMI by Gender, Overall Health for NHANES ages 21-79",
       x = "Body-mass index") +
  guides(fill = FALSE) +
  facet_grid(HealthGen ~ Gender)
```



### 3.10 Conclusions

This is just a small piece of the toolbox for visualizations that we'll create in this class. Many additional tools are on the way, but the main idea won't change. Using the `ggplot2` package, we can accomplish several critical tasks in creating a visualization, including:

- Identifying (and labeling) the axes and titles
- Identifying a type of `geom` to use, like a point, bar or histogram
- Changing fill, color, shape, size to facilitate comparisons
- Building “small multiples” of plots with faceting

Good data visualizations make it easy to see the data, and `ggplot2`'s tools make it relatively difficult to make a really bad graph.

# Chapter 4

## Data Structures and Types of Variables

### 4.1 Data require structure and context

**Descriptive statistics** are concerned with the presentation, organization and summary of data, as suggested in Norman and Streiner (2014). This includes various methods of organizing and graphing data to get an idea of what those data can tell us.

As Vittinghoff et al. (2012) suggest, the nature of the measurement determines how best to describe it statistically, and the main distinction is between **numerical** and **categorical** variables. Even this is a little tricky - plenty of data can have values that look like numerical values, but are just numerals serving as labels.

As Bock, Velleman, and De Veaux (2004) point out, the truly critical notion, of course, is that data values, no matter what kind, are useless without their contexts. The Five W's (Who, What [and in what units], When, Where, Why, and often How) are just as useful for establishing the context of data as they are in journalism. If you can't answer Who and What, in particular, you don't have any useful information.

In general, each row of a data frame corresponds to an individual (respondent, experimental unit, record, or observation) about whom some characteristics are gathered in columns (and these characteristics may be called variables, factors or data elements.) Every column / variable should have a name that indicates *what* it is measuring, and every row / observation should have a name that indicates *who* is being measured.

### 4.2 A New NHANES Adult Sample

In previous work, we spent some time with a sample from the National Health and Nutrition Examination. Now, by changing the value of the `set.seed` function which determines the starting place for the random sampling, and changing some other specifications, we'll generate a new sample describing 500 adult subjects who completed the 2011-12 version of the survey when they were between the ages of 21 and 64.

Note also that what is listed in the NHANES data frame as `Gender` should be more correctly referred to as `sex`. Sex is a biological feature of an individual, while `Gender` is a social construct. This is an important distinction, so I'll change the name of the variable. I'm also changing the names of three other variables, to create `Race`, `SBP` and `DBP`.

```
# library(NHANES) # NHANES package/library of functions, data  
nh_temp <- NHANES %>%
```

```

filter(SurveyYr == "2011_12") %>%
filter(Age >= 21 & Age < 65) %>%
mutate(Sex = Gender, Race = Race3, SBP = BPSysAve, DBP = BPDiaAve) %>%
select(ID, Sex, Age, Race, Education, BMI, SBP, DBP, Pulse, PhysActive, Smoke100, SleepTrouble, HealthGen)

set.seed(431002)
# use set.seed to ensure that we all get the same random sample

nh_adults <- sample_n(nh_temp, size = 500)

nh_adults

```

# A tibble: 500 x 13

	ID	Sex	Age	Race	Education	BMI	SBP	DBP	Pulse
1	64427	male	37	White	College Grad	36.5	111	72	56
2	63788	female	40	White	High School	18.2	115	74	102
3	66874	female	31	White	Some College	27.2	95	52	98
4	69734	male	26	White	College Grad	20.6	137	75	74
5	70409	male	44	White	High School	29.2	112	71	62
6	68961	female	64	White	College Grad	24.2	123	70	80
7	62616	female	37	Asian	8th Grade	19.3	109	73	82
8	70130	male	42	Black	High School	31.2	119	71	62
9	71218	male	33	White	College Grad	27.7	110	67	68
10	69181	female	37	White	8th Grade	25.0	114	74	82

# ... with 490 more rows, and 4 more variables: PhysActive <fctr>,  
# Smoke100 <fctr>, SleepTrouble <fctr>, HealthGen <fctr>

The data consist of 500 rows (observations) on 13 variables (columns). Essentially, we have 13 pieces of information on each of 500 adult NHANES subjects who were included in the 2011-12 panel.

#### 4.2.1 Summarizing the Data's Structure

We can identify the number of rows and columns in a data frame or tibble with the `dim` function.

```
dim(nh_adults)
```

```
[1] 500 13
```

The `str` function provides a lot of information about the structure of a data frame or tibble.

```
str(nh_adults)
```

```

Classes 'tbl_df', 'tbl' and 'data.frame': 500 obs. of 13 variables:
 $ ID      : int  64427 63788 66874 69734 70409 ...
 $ Sex     : Factor w/ 2 levels "female","male": 2 1 1 2 2 1 1 2 2 1 ...
 $ Age     : int  37 40 31 26 44 64 37 42 33 37 ...
 $ Race    : Factor w/ 6 levels "Asian","Black",...
 $ Education: Factor w/ 5 levels "8th Grade","9 - 11th Grade",...
 $ BMI     : num  36.5 18.2 27.2 20.6 29.2 ...
 $ SBP     : int  111 115 95 137 112 123 109 119 110 114 ...
 $ DBP     : int  72 74 52 75 71 70 73 71 67 74 ...
 $ Pulse   : int  56 102 98 74 62 80 82 62 68 82 ...
 $ PhysActive: Factor w/ 2 levels "No","Yes": 2 2 2 2 2 2 1 1 2 2 ...
 $ Smoke100 : Factor w/ 2 levels "No","Yes": 1 2 1 1 2 2 1 1 1 2 ...

```

```
$ SleepTrouble: Factor w/ 2 levels "No","Yes": 1 2 1 1 1 1 1 1 1 2 ...
$ HealthGen : Factor w/ 5 levels "Excellent","Vgood",...: 2 3 3 1 3 2 3 3 3 2 ...
```

To see the first few observations, use `head`, and to see the last few, try `tail`...

```
tail(nh_adults, 5) # shows the last five observations in the data set
```

```
# A tibble: 5 x 13
  ID     Sex   Age   Race    Education   BMI   SBP   DBP Pulse
  <int> <fctr> <int> <fctr>      <fctr> <dbl> <int> <int> <int>
1 69692 male    50 Black  9 - 11th Grade  22.7   132    82    60
2 66472 male    61 White   Some College  41.3   141    77    62
3 71456 male    21 Mexican 9 - 11th Grade  26.7   113    66    78
4 71420 female  54 Mexican 9 - 11th Grade  32.5   126    69    68
5 63617 male    29 White   College Grad  23.2   105    72    76
# ... with 4 more variables: PhysActive <fctr>, Smoke100 <fctr>,
#   SleepTrouble <fctr>, HealthGen <fctr>
```

## 4.2.2 What are the variables?

The variables we have collected are described in the brief table below<sup>1</sup>.

Variable	Description	Sample Values
ID	a numerical code identifying the subject	64427, 63788
Sex	sex of subject (2 levels)	male, female
Age	age (years) at screening of subject	37, 40
Race	reported race of subject (6 levels)	White, Asian
Education	educational level of subject (5 levels)	College Grad, High School
BMI	body-mass index, in kg/m <sup>2</sup>	36.5, 18.2
SBP	systolic blood pressure in mm Hg	111, 115
DBP	diastolic blood pressure in mm Hg	72, 74
Pulse	60 second pulse rate in beats per minute	56, 102
PhysActive	Moderate or vigorous-intensity sports?	Yes, No
Smoke100	Smoked at least 100 cigarettes lifetime?	Yes, No
SleepTrouble	Told a doctor they have trouble sleeping?	Yes, No
HealthGen	Self-report general health rating (5 lev.)	Vgood, Good

The levels for the multi-categorical variables are:

- **Race:** Mexican, Hispanic, White, Black, Asian, or Other.
- **Education:** 8th Grade, 9 - 11th Grade, High School, Some College, or College Grad.
- **HealthGen:** Excellent, Vgood, Good, Fair or Poor.

## 4.3 Types of Variables

### 4.3.1 Quantitative Variables

Variables recorded in numbers that we use as numbers are called **quantitative**. Familiar examples include incomes, heights, weights, ages, distances, times, and counts. All quantitative variables have measurement

<sup>1</sup>Descriptions are adapted from the ?NHANES help file. Remember that what NHANES lists as Gender is captured here as Sex, and similarly Race3, BPSysAve and BPDiaAve from NHANES are here listed as Race, SBP and DBP.

units, which tell you how the quantitative variable was measured. Without units (like miles per hour, angstroms, yen or degrees Celsius) the values of a quantitative variable have no meaning.

- It does little good to be promised a salary of 80,000 a year if you don't know whether it will be paid in Euros, dollars, yen or Estonian kroon.
- You might be surprised to see someone whose age is 72 listed in a database on childhood diseases until you find out that age is measured in months.
- Often just seeking the units can reveal a variable whose definition is challenging - just how do we measure "friendliness", or "success," for example.
- Quantitative variables may also be classified by whether they are **continuous** or can only take on a **discrete** set of values. Continuous data may take on any value, within a defined range. Suppose we are measuring height. While height is really continuous, our measuring stick usually only lets us measure with a certain degree of precision. If our measurements are only trustworthy to the nearest centimeter with the ruler we have, we might describe them as discrete measures. But we could always get a more precise ruler. The measurement divisions we make in moving from a continuous concept to a discrete measurement are usually fairly arbitrary. Another way to think of this, if you enjoy music, is that, as suggested in Norman and Streiner (2014), a piano is a *discrete* instrument, but a violin is a *continuous* one, enabling finer distinctions between notes than the piano is capable of making. Sometimes the distinction between continuous and discrete is important, but usually, it's not.
  - The `nh_adults` data includes several quantitative variables, specifically Age, BMI, SBP, DBP and Pulse.
  - We know these are quantitative because they have units: Age in years, BMI in kg/m<sup>2</sup>, the BP measurements in mm Hg, and Pulse in beats per minute.
  - Depending on the context, we would likely treat most of these as *discrete* given that measurements are fairly crude (this is certainly true for Age, measured in years) although BMI is probably *continuous* in most settings, even though it is a function of two other measures (Height and Weight) which are rounded off to integer numbers of centimeters and kilograms, respectively.
- It is also possible to separate out quantitative variables into **ratio** variables or **interval** variables. An interval variable has equal distances between values, but the zero point is arbitrary. A ratio variable has equal intervals between values, and a meaningful zero point. For example, weight is an example of a ratio variable, while IQ is an example of an interval variable. We all know what zero weight is. An intelligence score like IQ is a different matter. We say that the average IQ is 100, but that's only by convention. We could just as easily have decided to add 400 to every IQ value and make the average 500 instead. Because IQ's intervals are equal, the difference between an IQ of 70 and an IQ of 80 is the same as the difference between 120 and 130. However, an IQ of 100 is not twice as high as an IQ of 50. The point is that if the zero point is artificial and moveable, then the differences between numbers are meaningful but the ratios between them are not. On the other hand, most lab test values are ratio variables, as are physical characteristics like height and weight. A person who weighs 100 kg is twice as heavy as one who weighs 50 kg; even when we convert kg to pounds, this is still true. For the most part, we can treat and analyze interval or ratio variables the same way.
  - Each of the quantitative variables in our `nh_adults` data can be thought of as ratio variables.
  - Quantitative variables lend themselves to many of the summaries we will discuss, like means, quantiles, and our various measures of spread, like the standard deviation or inter-quartile range. They also have at least a chance to follow the Normal distribution.

### 4.3.2 Qualitative (Categorical) Variables

**Qualitative** or categorical variables consist of names of categories. These names may be numerical, but the numbers (or names) are simply codes to identify the groups or categories into which the individuals are divided. Categorical variables with two categories, like yes or no, up or down, or, more generally, 1 and 0,

are called **binary** variables. Those with more than two-categories are sometimes called **multi-categorical** variables.

- When the categories included in a variable are merely names, and come in no particular order, we sometimes call them **nominal** variables. The most important summary of such a variable is usually a table of frequencies, and the mode becomes an important single summary, while the mean and median are essentially useless.
  - In the `nh_adults` data, Race is clearly a nominal variable with multiple unordered categories.
- The alternative categorical variable (where order matters) is called **ordinal**, and includes variables that are sometimes thought of as falling right in between quantitative and qualitative variables.
  - Examples of ordinal multi-categorical variables in the `nh_adults` data include the Education and HealthGen variables.
  - Answers to questions like “How is your overall physical health?” with available responses Excellent, Very Good, Good, Fair or Poor, which are often coded as 1-5, certainly provide a perceived *order*, but a group of people with average health status 4 (Very Good) is not necessarily twice as healthy as a group with average health status of 2 (Fair).
- Sometimes we treat the values from ordinal variables as sufficiently scaled to permit us to use quantitative approaches like means, quantiles, and standard deviations to summarize and model the results, and at other times, we’ll treat ordinal variables as if they were nominal, with tables and percentages our primary tools.
- Note that all binary variables may be treated as ordinal, or nominal.
  - Binary variables in the `nh_adults` data include Sex, PhysActive, Smoke100, SleepTrouble. Each can be thought of as either ordinal or nominal.

Lots of variables may be treated as either quantitative or qualitative, depending on how we use them. For instance, we usually think of age as a quantitative variable, but if we simply use age to make the distinction between “child” and “adult” then we are using it to describe categorical information. Just because your variable’s values are numbers, don’t assume that the information provided is quantitative.



# Chapter 5

## Summarizing Quantitative Variables

Most numerical summaries that might be new to you are applied most appropriately to quantitative variables. The measures that will interest us relate to:

- the **center** of our distribution,
- the **spread** of our distribution, and
- the **shape** of our distribution.

### 5.1 The **summary** function for Quantitative data

R provides a small sampling of numerical summaries with the **summary** function, for instance.

```
nh_adults %>%
  select(Age, BMI, SBP, DBP, Pulse) %>%
  summary()
```

	Age	BMI	SBP	DBP	Pulse
Min.	:21.0	Min. :17.8	Min. : 84	Min. : 19.0	Min. : 46
1st Qu.	:31.0	1st Qu.:24.2	1st Qu.:109	1st Qu.: 65.0	1st Qu.: 64
Median	:42.0	Median :27.7	Median :118	Median : 72.0	Median : 72
Mean	:42.1	Mean :28.7	Mean :119	Mean : 72.2	Mean : 73
3rd Qu.	:53.0	3rd Qu.:32.1	3rd Qu.:127	3rd Qu.: 79.0	3rd Qu.: 80
Max.	:64.0	Max. :69.0	Max. :202	Max. :105.0	Max. :120
NA's	:3	NA's :15	NA's :15	NA's :15	NA's :15

This basic summary includes a set of five **quantiles**<sup>1</sup>, plus the sample's **mean**.

- **Min.** = the **minimum** value for each variable, so, for example, the youngest subject's Age was 21.
- **1st Qu.** = the **first quartile** (25<sup>th</sup> percentile) for each variable - for example, 25% of the subjects were Age 31 or younger.
- **Median** = the **median** (50<sup>th</sup> percentile) - half of the subjects were Age 42 or younger.
- **Mean** = the **mean**, usually what one means by an *average* - the sum of the Ages divided by 500 is 42.1,
- **3rd Qu.** = the **third quartile** (75<sup>th</sup> percentile) - 25% of the subjects were Age 53 or older.
- **Max.** = the **maximum** value for each variable, so the oldest subject was Age 64.

The summary also specifies the number of missing values for each variable. Here, we are missing 3 of the BMI values, for example.

<sup>1</sup>The quantiles (sometimes referred to as percentiles) can also be summarised with a boxplot.

## 5.2 Measuring the Center of a Distribution

### 5.2.1 The Mean and The Median

The **mean** and **median** are the most commonly used measures of the center of a distribution for a quantitative variable. The median is the more generally useful value, as it is relevant even if the data have a shape that is not symmetric. We might also collect the **sum** of the observations, and the **count** of the number of observations, usually symbolized with  $n$ .

For variables without missing values, like `Age`, this is pretty straightforward.

```
nh_adults %>%
  summarise(n = n(), Mean = mean(Age), Median = median(Age), Sum = sum(Age))
```

```
# A tibble: 1 x 4
  n   Mean Median   Sum
  <int> <dbl> <dbl> <int>
1 500  42.1    42 21051
```

And again, the Mean is just the Sum (21051), divided by the number of non-missing values of Age (500), or 42.102.

The Median is the middle value when the data are sorted in order. When we have an odd number of values, this is sufficient. When we have an even number, as in this case, we take the mean of the two middle values. We could sort and list all 500 Ages, if we wanted to do so.

```
nh_adults %>% select(Age) %>%
  arrange(Age)
```

```
# A tibble: 500 x 1
  Age
  <int>
1 21
2 21
3 21
4 21
5 21
6 21
7 21
8 21
9 21
10 21
# ... with 490 more rows
```

But this data set figures we don't want to output more than 10 observations to a table like this.

If we really want to see all of the data, we can use `View(nh_adults)` to get a spreadsheet-style presentation, or use the `sort` command...

```
sort(nh_adults$Age)
```

```
[1] 21 21 21 21 21 21 21 21 21 21 21 21 21 22 22 22 22 22 22 22 22 22 22 22 23
[24] 23 23 23 23 23 23 23 23 23 23 23 23 23 24 24 24 24 24 24 24 24 24 24 24 24
[47] 24 25 25 25 25 25 25 25 25 25 25 25 25 26 26 26 26 26 26 26 26 26 26 26 26
[70] 26 26 27 27 27 27 27 27 27 27 27 27 27 28 28 28 28 28 28 28 28 28 28 28 28
[93] 28 28 28 28 28 28 28 28 29 29 29 29 29 29 29 29 29 29 29 29 30 30 30 30
[116] 30 30 30 30 30 30 30 31 31 31 31 31 31 31 31 31 31 31 31 32 32 32 32
[139] 32 32 32 32 32 32 33 33 33 33 33 33 33 33 33 33 33 34 34 34 34
```

```
[162] 34 34 34 34 35 35 35 36 36 36 36 36 36 36 36 36 36 36 36 36 36 37 37 37 37 37 37
[185] 37 37 37 37 37 37 37 37 37 38 38 38 38 38 38 38 38 38 38 38 39 39 39 39
[208] 39 39 39 39 39 39 39 39 39 40 40 40 40 40 40 40 40 40 40 40 41 41 41
[231] 41 41 41 41 41 41 42 42 42 42 42 42 42 42 42 42 42 42 42 42 42 42 42 43
[254] 43 43 43 43 43 43 43 43 43 44 44 44 44 44 44 44 44 44 44 44 44 44 44 45
[277] 45 45 45 45 45 45 46 46 46 46 46 46 46 46 46 46 46 46 46 46 46 47 47 47
[300] 47 47 47 47 47 47 48 48 48 48 48 48 48 48 48 48 48 49 49 49 49 49 49 49
[323] 49 49 49 49 49 49 49 49 50 50 50 50 50 50 50 50 50 50 50 50 50 50 50 50
[346] 50 50 50 50 51 51 51 51 51 51 51 51 51 51 51 52 52 52 52 52 52 52 52
[369] 52 52 52 53 53 53 53 53 53 53 53 53 53 53 54 54 54 54 54 54 54 54 54
[392] 54 54 54 54 55 55 55 55 55 55 56 56 56 56 56 56 56 56 56 56 56 56 56 56
[415] 56 56 56 56 56 56 57 57 57 57 57 57 57 57 57 57 57 57 57 57 58 58 58 58
[438] 58 58 58 58 58 58 59 59 59 59 59 59 59 59 59 59 59 59 60 60 60 60 60 60
[461] 60 60 60 60 60 60 61 61 61 61 61 61 61 61 61 61 61 61 61 62 62 62 62 62
[484] 62 62 62 63 63 63 63 63 64 64 64 64 64 64 64 64 64 64 64 64 64 64 64
```

Again, to find the median, we would take the mean of the middle two observations in this sorted data set. That would be the 250<sup>th</sup> and 251<sup>st</sup> largest Ages.

```
sort(nh_adults$Age)[250:251]
```

```
[1] 42 42
```

## 5.2.2 Dealing with Missingness

When calculating a mean, you may be tempted to try something like this...

```
nh_adults %>%
  summarise(mean(Pulse), median(Pulse))

# A tibble: 1 x 2
`mean(Pulse)` `median(Pulse)`
<dbl>          <int>
1           NA            NA
```

This fails because we have some missing values in the Pulse data. We can address this by either omitting the data with missing values before we run the summarise function, or tell the mean and median summary functions to remove missing values<sup>2</sup>.

```
nh_adults %>%
  filter(complete.cases(Pulse)) %>%
  summarise(count = n(), mean(Pulse), median(Pulse))

# A tibble: 1 x 3
count `mean(Pulse)` `median(Pulse)`
<int>      <dbl>        <int>
1    485         73          72
```

Or, we could tell the summary functions themselves to remove NA values.

```
nh_adults %>%
  summarise(mean(Pulse, na.rm=TRUE), median(Pulse, na.rm=TRUE))

# A tibble: 1 x 2
`mean(Pulse, na.rm = TRUE)` `median(Pulse, na.rm = TRUE)`
<dbl>                      <int>
```

<sup>2</sup>We could also use !is.na in place of complete.cases to accomplish the same thing.

1

73

72

While we eventually discuss the importance of **imputation** when dealing with missing data, this doesn't apply to providing descriptive summaries of actual, observed values.

### 5.2.3 The Mode of a Quantitative Variable

One other less common measure of the center of a quantitative variable's distribution is its most frequently observed value, referred to as the **mode**. This measure is only appropriate for discrete variables, be they quantitative or categorical. To find the mode, we usually tabulate the data, and then sort by the counts of the numbers of observations.

```
nh_adults %>%
  group_by(Age) %>%
  summarise(count = n()) %>%
  arrange(desc(count))
```

```
# A tibble: 44 x 2
  Age   count
  <int> <int>
1    56     19
2    50     18
3    28     16
4    37     16
5    42     16
6    49     15
7    24     13
8    27     13
9    39     13
10   46     13
# ... with 34 more rows
```

Note the use of three different “verbs” in our function there - for more explanation of this strategy, visit Grolemund and Wickham (2017).

As an alternative, the **modeest** package's **mfv** function calculates the sample mode (or most frequent value).<sup>3</sup>

## 5.3 Measuring the Spread of a Distribution

Statistics is all about variation, so spread or dispersion is an important fundamental concept in statistics. Measures of spread like the inter-quartile range and range (maximum - minimum) can help us understand and compare data sets. If the values in the data are close to the center, the spread will be small. If many of the values in the data are scattered far away from the center, the spread will be large.

### 5.3.1 The Range and the Interquartile Range (IQR)

The **range** of a quantitative variable is sometimes interpreted as the difference between the maximum and the minimum, even though R presents the actual minimum and maximum values when you ask for a range...

```
nh_adults %>%
  select(Age) %>%
  range()
```

---

<sup>3</sup>See the documentation for the **modeest** package's **mlv** function to look at other definitions of the mode.

```
[1] 21 64
```

And, for a variable with missing values, we can use...

```
nh_adults %>%
  select(BMI) %>%
  range(., na.rm=TRUE)
```

```
[1] 17.8 69.0
```

A more interesting and useful statistic is the **inter-quartile range**, or IQR, which is the range of the middle half of the distribution, calculated by subtracting the 25<sup>th</sup> percentile value from the 75<sup>th</sup> percentile value.

```
nh_adults %>%
  summarise(IQR(Age), quantile(Age, 0.25), quantile(Age, 0.75))
```

```
# A tibble: 1 x 3
`IQR(Age)` `quantile(Age, 0.25)` `quantile(Age, 0.75)`
<dbl>          <dbl>          <dbl>
1        22            31            53
```

We can calculate the range and IQR nicely from the summary information on quantiles, of course:

```
nh_adults %>%
  select(Age, BMI, SBP, DBP, Pulse) %>%
  summary()
```

	Age	BMI	SBP	DBP	Pulse
Min.	:21.0	Min. :17.8	Min. : 84	Min. : 19.0	Min. : 46
1st Qu.	:31.0	1st Qu.:24.2	1st Qu.:109	1st Qu.: 65.0	1st Qu.: 64
Median	:42.0	Median :27.7	Median :118	Median : 72.0	Median : 72
Mean	:42.1	Mean :28.7	Mean :119	Mean : 72.2	Mean : 73
3rd Qu.	:53.0	3rd Qu.:32.1	3rd Qu.:127	3rd Qu.: 79.0	3rd Qu.: 80
Max.	:64.0	Max. :69.0	Max. :202	Max. :105.0	Max. :120
NA's	:3	NA's :15	NA's :15	NA's :15	NA's :15

### 5.3.2 The Variance and the Standard Deviation

The IQR is always a reasonable summary of spread, just as the median is always a reasonable summary of the center of a distribution. Yet, most people are inclined to summarise a batch of data using two numbers: the **mean** and the **standard deviation**. This is really only a sensible thing to do if you are willing to assume the data follow a Normal distribution: a bell-shaped, symmetric distribution without substantial outliers.

But **most data do not (even approximately) follow a Normal distribution**. Summarizing by the median and quartiles (25th and 75th percentiles) is much more robust, explaining R's emphasis on them.

### 5.3.3 Obtaining the Variance and Standard Deviation in R

Here are the variances of the quantitative variables in the `nh_adults` data. Note the need to include `na.rm = TRUE` to deal with the missing values in some variables.

```
nh_adults %>%
  select(Age, BMI, SBP, DBP, Pulse) %>%
  summarise_all(var, na.rm = TRUE)
```

```
# A tibble: 1 x 5
  Age    BMI    SBP    DBP  Pulse
  <dbl> <dbl> <dbl> <dbl> <dbl>
```

```
<dbl> <dbl> <dbl> <dbl> <dbl>
1   157   42.1   234   117   132
```

And here are the standard deviations of those same variables.

```
nh_adults %>%
  select(Age, BMI, SBP, DBP, Pulse) %>%
  summarise_all(sd, na.rm = TRUE)

# A tibble: 1 x 5
  Age    BMI   SBP   DBP Pulse
  <dbl> <dbl> <dbl> <dbl>
1 12.5  6.49 15.3 10.8 11.5
```

### 5.3.4 Defining the Variance and Standard Deviation

Bock, Velleman, and De Veaux (2004) have lots of useful thoughts here, which are lightly edited here.

In thinking about spread, we might consider how far each data value is from the mean. Such a difference is called a *deviation*. We could just average the deviations, but the positive and negative differences always cancel out, leaving an average deviation of zero, so that's not helpful. Instead, we *square* each deviation to obtain non-negative values, and to emphasize larger differences. When we add up these squared deviations and find their mean (almost), this yields the **variance**.

$$\text{Variance} = s^2 = \frac{\sum(y - \bar{y})^2}{n - 1}$$

Why almost? It would be the mean of the squared deviations only if we divided the sum by  $n$ , but instead we divide by  $n - 1$  because doing so produces an estimate of the true (population) variance that is *unbiased*<sup>4</sup>. If you're looking for a more intuitive explanation, this Stack Exchange link awaits your attention.

- To return to the original units of measurement, we take the square root of  $s^2$ , and instead work with  $s$ , the **standard deviation**.

$$\text{Standard Deviation} = s = \sqrt{\frac{\sum(y - \bar{y})^2}{n - 1}}$$

### 5.3.5 Empirical Rule Interpretation of the Standard Deviation

For a set of measurements that follow a Normal distribution, the interval:

- Mean  $\pm$  Standard Deviation contains approximately 68% of the measurements;
- Mean  $\pm$  2(Standard Deviation) contains approximately 95% of the measurements;
- Mean  $\pm$  3(Standard Deviation) contains approximately all (99.7%) of the measurements.

We often refer to the population or process mean of a distribution with  $\mu$  and the standard deviation with  $\sigma$ , leading to the Figure below.

But if the data are not from an approximately Normal distribution, then this Empirical Rule is less helpful.

---

<sup>4</sup>When we divide by  $n-1$  as we calculate the sample variance, the average of the sample variances for all possible samples is equal to the population variance. If we instead divided by  $n$ , the average sample variance across all possible samples would be a little smaller than the population variance.

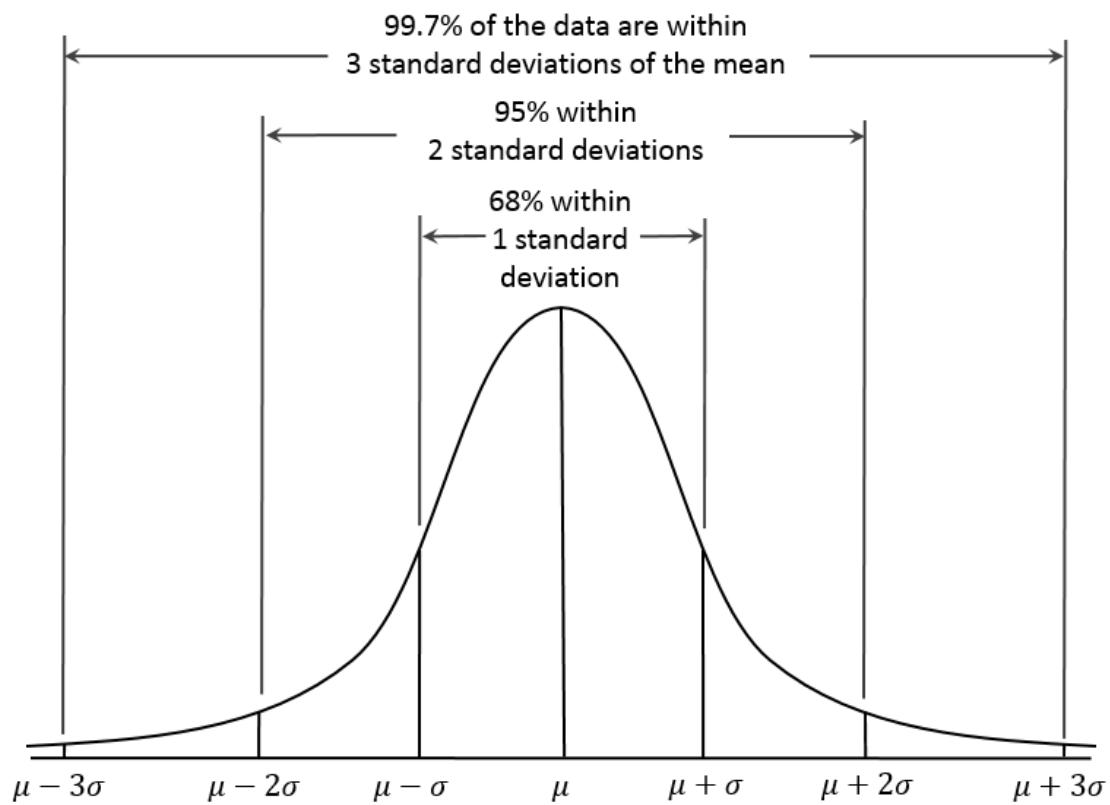


Figure 5.1: The Normal Distribution and the Empirical Rule

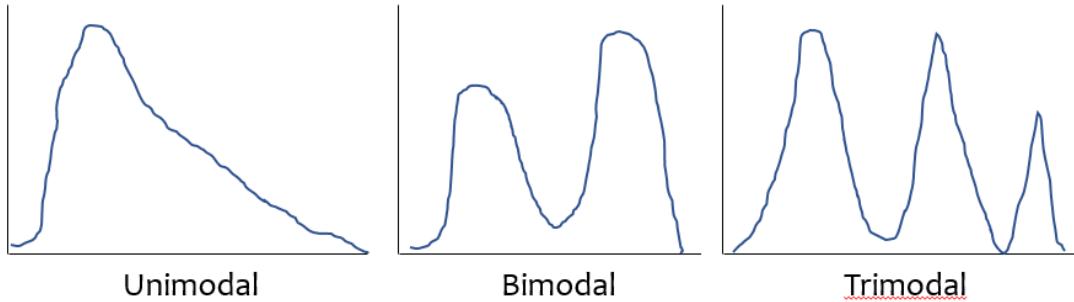


Figure 5.2: Unimodal and Multimodal Sketches

### 5.3.6 Chebyshev's Inequality: One Interpretation of the Standard Deviation

Chebyshev's Inequality tells us that for any distribution, regardless of its relationship to a Normal distribution, no more than  $1/k^2$  of the distribution's values can lie more than  $k$  standard deviations from the mean. This implies, for instance, that for **any** distribution, at least 75% of the values must lie within two standard deviations of the mean, and at least 89% must lie within three standard deviations of the mean.

Again, most data sets do not follow a Normal distribution. We'll return to this notion soon. But first, let's try to draw some pictures that let us get a better understanding of the distribution of our data.

## 5.4 Measuring the Shape of a Distribution

When considering the shape of a distribution, one is often interested in three key points.

- The number of modes in the distribution, which I always assess through plotting the data.
- The **skewness**, or symmetry that is present, which I typically assess by looking at a plot of the distribution of the data, but if required to, will summarise with a non-parametric measure of **skewness**.
- The **kurtosis**, or heavy-tailedness (outlier-proneness) that is present, usually in comparison to a Normal distribution. Again, this is something I nearly inevitably assess graphically, but there are measures.

A Normal distribution has a single mode, is symmetric and, naturally, is neither heavy-tailed nor light-tailed as compared to a Normal distribution (we call this mesokurtic).

### 5.4.1 Multimodal vs. Unimodal distributions

A unimodal distribution, on some level, is straightforward. It is a distribution with a single mode, or “peak” in the distribution. Such a distribution may be skewed or symmetric, light-tailed or heavy-tailed. We usually describe as multimodal distributions like the two on the right below, which have multiple local maxima, even though they have just a single global maximum peak.

Truly multimodal distributions are usually described that way in terms of shape. For unimodal distributions, skewness and kurtosis become useful ideas.

### 5.4.2 Skew

Whether or not a distribution is approximately symmetric is an important consideration in describing its shape. Graphical assessments are always most useful in this setting, particularly for unimodal data. My favorite measure of skew, or skewness if the data have a single mode, is:

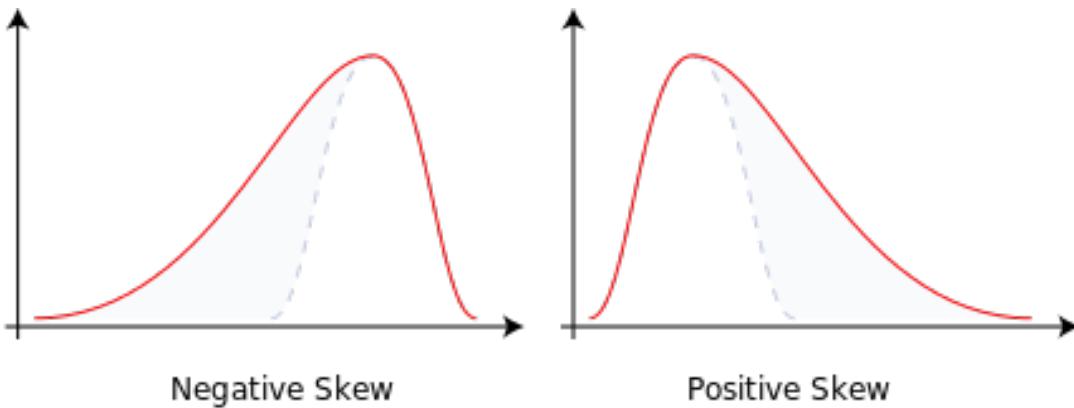


Figure 5.3: Negative (Left) Skew and Positive (Right) Skew

$$skew_1 = \frac{\text{mean} - \text{median}}{\text{standard deviation}}$$

- Symmetric distributions generally show values of  $skew_1$  near zero. If the distribution is actually symmetric, the mean should be equal to the median.
- Distributions with  $skew_1$  values above 0.2 in absolute value generally indicate meaningful skew.
- Positive skew (mean > median if the data are unimodal) is also referred to as *right skew*.
- Negative skew (mean < median if the data are unimodal) is referred to as *left skew*.

### 5.4.3 Kurtosis

When we have a unimodal distribution that is symmetric, we will often be interested in the behavior of the tails of the distribution, as compared to a Normal distribution with the same mean and standard deviation. High values of kurtosis measures (and there are several) indicate data which has extreme outliers, or is heavy-tailed.

- A mesokurtic distribution has similar tail behavior to what we would expect from a Normal distribution.
- A leptokurtic distribution is a thinner distribution, with lighter tails (fewer observations far from the center) than we'd expect from a Normal distribution.
- A platykurtic distribution is a flatter distribution, with heavier tails (more observations far from the center) than we'd expect from a Normal distribution.

Graphical tools are in most cases the best way to identify issues related to kurtosis.

## 5.5 More Detailed Numerical Summaries for Quantitative Variables

### 5.5.1 favstats in the mosaic package

The `favstats` function adds the standard deviation, and counts of overall and missing observations to our usual `summary` for a continuous variable. Let's look at systolic blood pressure, because we haven't yet.

```
mosaic::favstats(~ SBP, data = nh_adults)
```

min	Q1	median	Q3	max	mean	sd	n	missing
84	109	118	127	202	119	15.3	485	15

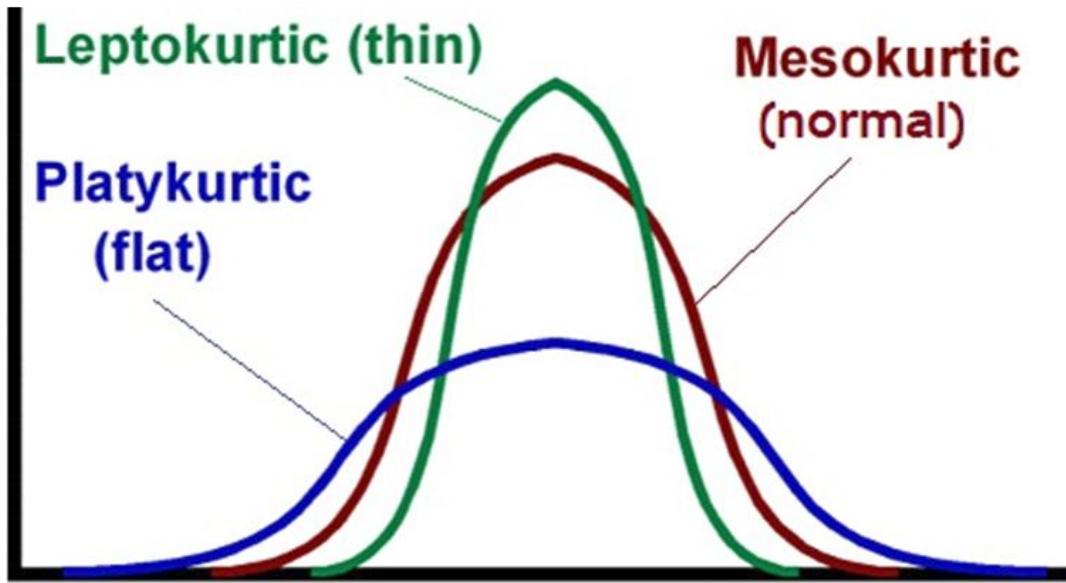


Figure 5.4: The Impact of Kurtosis

We could, of course, duplicate these results with a rather lengthy set of `summarise` pieces...

```
nh_adults %>%
  filter(complete.cases(SBP)) %>%
  summarise(min = min(SBP), Q1 = quantile(SBP, 0.25), median = median(SBP),
            Q3 = quantile(SBP, 0.75), max = max(SBP),
            mean = mean(SBP), sd = sd(SBP), n = n(), missing = sum(is.na(SBP)))
```

```
# A tibble: 1 x 9
  min    Q1 median    Q3   max   mean     sd      n missing
  <dbl> <dbl> <int> <dbl> <dbl> <dbl> <dbl> <int>
1    84    109    118    127   202   119   15.3    485      0
```

The somewhat unusual structure of `favstats` (complete with an easy to forget ~) is actually helpful. It allows you to look at some interesting grouping approaches, like this:

```
mosaic::favstats(SBP ~ Education, data = nh_adults)
```

	Education	min	Q1	median	Q3	max	mean	sd	n	missing
1	8th Grade	95	109	122	126	147	119	14.1	21	3
2	9 - 11th Grade	100	111	115	126	152	118	12.0	57	0
3	High School	89	109	120	129	202	121	19.7	78	3
4	Some College	85	110	118	128	163	119	14.6	149	4
5	College Grad	84	108	116	124	172	117	14.7	180	5

Of course, we could accomplish the same comparison with `dplyr` commands, too, but the `favstats` approach has much to offer.

```
nh_adults %>%
  filter(complete.cases(SBP, Education)) %>%
  group_by(Education) %>%
  summarise(min = min(SBP), Q1 = quantile(SBP, 0.25), median = median(SBP),
            Q3 = quantile(SBP, 0.75), max = max(SBP),
```

```
mean = mean(SBP), sd = sd(SBP), n = n(), missing = sum(is.na(SBP)))
```

	Education	min	Q1	median	Q3	max	mean	sd	n	missing
	<fctr>	<dbl>	<dbl>	<dbl>	<dbl>	<dbl>	<dbl>	<dbl>	<int>	<int>
1	8th Grade	95	109	122	126	147	119	14.1	21	0
2	9 - 11th Grade	100	111	115	126	152	118	12.0	57	0
3	High School	89	109	120	129	202	121	19.7	78	0
4	Some College	85	110	118	128	163	119	14.6	149	0
5	College Grad	84	108	116	124	172	117	14.7	180	0

### 5.5.2 describe in the psych package

The psych package has a more detailed list of numerical summaries for quantitative variables that lets us look at a group of observations at once.

```
psych::describe(nh_adults %>% select(Age, BMI, SBP, DBP, Pulse))
```

	vars	n	mean	sd	median	trimmed	mad	min	max	range	skew
Age	1	500	42.1	12.54	42.0	42.1	16.31	21.0	64	43.0	-0.03
BMI	2	497	28.7	6.49	27.7	28.1	5.78	17.8	69	51.2	1.33
SBP	3	485	118.6	15.30	118.0	117.8	13.34	84.0	202	118.0	1.00
DBP	4	485	72.2	10.83	72.0	72.1	10.38	19.0	105	86.0	-0.05
Pulse	5	485	73.0	11.47	72.0	72.5	11.86	46.0	120	74.0	0.46
			kurtosis	se							
Age			-1.23	0.56							
BMI			4.15	0.29							
SBP			3.44	0.69							
DBP			1.07	0.49							
Pulse			0.45	0.52							

The additional statistics presented here are:

- **trimmed** = a trimmed mean (by default in this function, this removes the top and bottom 10% from the data, then computes the mean of the remaining values - the middle 80% of the full data set.)
- **mad** = the median absolute deviation (from the median), which can be used in a manner similar to the standard deviation or IQR to measure spread.
  - If the data are  $Y_1, Y_2, \dots, Y_n$ , then the **mad** is defined as  $\text{median}(|Y_i - \text{median}(Y_i)|)$ .
  - To find the **mad** for a set of numbers, find the median, subtract the median from each value and find the absolute value of that difference, and then find the median of those absolute differences.
  - For non-normal data with a skewed shape but tails well approximated by the Normal, the **mad** is likely to be a better (more robust) estimate of the spread than is the standard deviation.
- a measure of **skew**, which refers to how much asymmetry is present in the shape of the distribution. The measure is not the same as the *nonparametric skew* measure that we will usually prefer. The [Wikipedia page on skewness][<https://en.wikipedia.org/wiki/Skewness>] is very detailed.
- a measure of **kurtosis**, which refers to how outlier-prone, or heavy-tailed the shape of the distribution is, mainly as compared to a Normal distribution.
- **se** = the standard error of the sample mean, equal to the sample sd divided by the square root of the sample size.

### 5.5.3 describe in the Hmisc package

```
Hmisc::describe(nh_adults %>% select(Age, BMI, SBP, DBP, Pulse))
```

```
nh_adults %>% select(Age, BMI, SBP, DBP, Pulse)
```

```
5 Variables      500 Observations
```

---

#### Age

	n	missing	distinct	Info	Mean	Gmd	.05	.10
	500	0	44	0.999	42.1	14.48	23	25
	.25	.50	.75	.90	.95			
	31	42	53	59	61			

lowest : 21 22 23 24 25, highest: 60 61 62 63 64

---

#### BMI

	n	missing	distinct	Info	Mean	Gmd	.05	.10
	497	3	203	1	28.73	6.947	19.90	22.00
	.25	.50	.75	.90	.95			
	24.20	27.70	32.10	36.54	40.82			

lowest : 17.8 18.0 18.1 18.2 18.4, highest: 47.6 48.6 48.8 62.8 69.0

---

#### SBP

	n	missing	distinct	Info	Mean	Gmd	.05	.10
	485	15	71	0.999	118.6	16.51	96	101
	.25	.50	.75	.90	.95			
	109	118	127	137	143			

lowest : 84 85 86 89 91, highest: 163 167 168 172 202

---

#### DBP

	n	missing	distinct	Info	Mean	Gmd	.05	.10
	485	15	57	0.999	72.25	12.04	56	59
	.25	.50	.75	.90	.95			
	65	72	79	86	90			

lowest : 19 41 45 47 49, highest: 100 101 102 103 105

---

#### Pulse

	n	missing	distinct	Info	Mean	Gmd	.05	.10
	485	15	31	0.997	72.96	12.81	56	60
	.25	.50	.75	.90	.95			
	64	72	80	88	92			

lowest : 46 48 50 52 54, highest: 98 100 102 108 120

---

The `Hmisc` package's version of `describe` for a distribution of data presents three new ideas, in addition to a more comprehensive list of quartiles (the 5<sup>th</sup>, 10<sup>th</sup>, 25<sup>th</sup>, 50<sup>th</sup>, 75<sup>th</sup>, 90<sup>th</sup> and 95<sup>th</sup> are shown) and the lowest and highest few observations. These are:

- **distinct** - the number of different values observed in the data.
- **Info** - a measure of how “continuous” the variable is, related to how many “ties” there are in the data, with Info taking a higher value (closer to its maximum of one) if the data are more continuous.
- **Gmd** - the Gini mean difference - a robust measure of spread that is calculated as the mean absolute difference between any pairs of observations. Larger values of Gmd indicate more spread-out distributions.

# Chapter 6

## Summarizing Categorical Variables

Summarizing categorical variables numerically is mostly about building tables, and calculating percentages or proportions. We'll save our discussion of modeling categorical data for later. Recall that in the `nh_adults` data set we built in Section (@ref(createnh\_adults)), we had the following categorical variables. The number of levels indicates the number of possible categories for each categorical variable.

Variable	Description	Levels	Type
Sex	sex of subject	2	binary
Race	subject's race	6	nominal
Education	subject's educational level	5	ordinal
PhysActive	Participates in sports?	2	binary
Smoke100	Smoked 100+ cigarettes?	2	binary
SleepTrouble	Trouble sleeping?	2	binary
HealthGen	Self-report health	5	ordinal

### 6.1 The `summary` function for Categorical data

When R recognizes a variable as categorical, it stores it as a *factor*. Such variables get special treatment from the `summary` function, in particular a table of available values (so long as there aren't too many.)

```
nh_adults %>%
```

```
  select(Sex, Race, Education, PhysActive, Smoke100, SleepTrouble, HealthGen) %>%
  summary()
```

```
Sex          Race           Education      PhysActive  Smoke100
female:253   Asian    : 29   8th Grade    : 24   No :225     No :289
male  :247    Black    : 57   9 - 11th Grade: 57   Yes:275    Yes:211
              Hispanic: 39   High School  : 81
              Mexican : 43   Some College: 153
              White   :322   College Grad: 185
              Other   : 10
SleepTrouble  HealthGen
No :362       Excellent: 51
Yes:138      Vgood    :153
              Good     :172
              Fair     : 71
              Poor    :  7
```

```
NA's      : 46
```

## 6.2 Tables to describe One Categorical Variable

Suppose we build a table to describe the `HealthGen` distribution.

```
nh_adults %>%
  select(HealthGen) %>%
  table(., useNA = "ifany")
```

	Excellent	Vgood	Good	Fair	Poor	<NA>
	51	153	172	71	7	46

The main tools we have for augmenting tables are:

- adding in marginal totals, and
- working with proportions/percentages.

What if we want to add a total count?

```
nh_adults %>%
  select(HealthGen) %>%
  table(., useNA = "ifany") %>%
  addmargins()
```

	Excellent	Vgood	Good	Fair	Poor	<NA>	Sum
	51	153	172	71	7	46	500

What if we want to leave out the missing responses?

```
nh_adults %>%
  select(HealthGen) %>%
  table(., useNA = "no") %>%
  addmargins()
```

	Excellent	Vgood	Good	Fair	Poor	Sum
	51	153	172	71	7	454

Let's put the missing values back in, but now calculate proportions instead. Since the total will just be 1.0, we'll leave that out.

```
nh_adults %>%
  select(HealthGen) %>%
  table(., useNA = "ifany") %>%
  prop.table()
```

	Excellent	Vgood	Good	Fair	Poor	<NA>
	0.102	0.306	0.344	0.142	0.014	0.092

Now, we'll calculate percentages by multiplying the proportions by 100.

```
nh_adults %>%
  select(HealthGen) %>%
  table(., useNA = "ifany") %>%
```

```
prop.table() %>%
  "*" (100)
```

	Excellent	Vgood	Good	Fair	Poor	<NA>
	10.2	30.6	34.4	14.2	1.4	9.2

## 6.3 The Mode of a Categorical Variable

A common measure applied to a categorical variable is to identify the mode, the most frequently observed value. To find the mode for variables with lots of categories (so that the `summary` may not be sufficient), we usually tabulate the data, and then sort by the counts of the numbers of observations, as we did with discrete quantitative variables.

```
nh_adults %>%
  group_by(HealthGen) %>%
  summarise(count = n()) %>%
  arrange(desc(count))
```

```
# A tibble: 6 x 2
  HealthGen count
  <fctr> <int>
1 Good      172
2 Vgood     153
3 Fair       71
4 Excellent  51
5 <NA>        46
6 Poor       7
```

## 6.4 `describe` in the `Hmisc` package

```
Hmisc::describe(nh_adults %>%
  select(Sex, Race, Education, PhysActive,
  Smoke100, SleepTrouble, HealthGen))

nh_adults %>% select(Sex, Race, Education, PhysActive, Smoke100, SleepTrouble, HealthGen)

7 Variables     500 Observations
-----
Sex
  n  missing distinct
  500      0       2

Value    female   male
Frequency 253    247
Proportion 0.506  0.494
-----
Race
  n  missing distinct
  500      0       6
```

Value	Asian	Black	Hispanic	Mexican	White	Other
Frequency	29	57	39	43	322	10
Proportion	0.058	0.114	0.078	0.086	0.644	0.020

## Education

n	missing	distinct
500	0	5

Value	8th Grade	9 - 11th Grade	High School	Some College
Frequency	24	57	81	153
Proportion	0.048	0.114	0.162	0.306

Value	College Grad
Frequency	185
Proportion	0.370

## PhysActive

n	missing	distinct
500	0	2

Value	No	Yes
Frequency	225	275
Proportion	0.45	0.55

## Smoke100

n	missing	distinct
500	0	2

Value	No	Yes
Frequency	289	211
Proportion	0.578	0.422

## SleepTrouble

n	missing	distinct
500	0	2

Value	No	Yes
Frequency	362	138
Proportion	0.724	0.276

## HealthGen

n	missing	distinct
454	46	5

Value	Excellent	Vgood	Good	Fair	Poor
Frequency	51	153	172	71	7
Proportion	0.112	0.337	0.379	0.156	0.015

## 6.5 Cross-Tabulations

It is very common for us to want to describe the association of one categorical variable with another. For instance, is there a relationship between Education and SleepTrouble in these data?

```
nh_adults %>%
  select(Education, SleepTrouble) %>%
  table() %>%
  addmargins()
```

		SleepTrouble		Sum
Education		No	Yes	
8th Grade		15	9	24
9 - 11th Grade		40	17	57
High School		67	14	81
Some College		107	46	153
College Grad		133	52	185
Sum		362	138	500

To get row percentages, we can use:

```
nh_adults %>%
  select(Education, SleepTrouble) %>%
  table() %>%
  prop.table(., 1) %>%
  "*"(100)
```

		SleepTrouble	
Education		No	Yes
8th Grade		62.5	37.5
9 - 11th Grade		70.2	29.8
High School		82.7	17.3
Some College		69.9	30.1
College Grad		71.9	28.1

For column percentages, we use 2 instead of 1 in the `prop.table` function. Here, we'll also round off to two decimal places:

```
nh_adults %>%
  select(Education, SleepTrouble) %>%
  table() %>%
  prop.table(., 2) %>%
  "*"(100) %>%
  round(., 2)
```

		SleepTrouble	
Education		No	Yes
8th Grade		4.14	6.52
9 - 11th Grade		11.05	12.32
High School		18.51	10.14
Some College		29.56	33.33
College Grad		36.74	37.68

Here's another approach, to look at the cross-classification of Race and HealthGen:

```
xtabs(~ Race + HealthGen, data = nh_adults)
```

HealthGen

Race	Excellent	Vgood	Good	Fair	Poor
Asian	4	7	9	2	1
Black	7	11	16	11	2
Hispanic	1	9	18	8	0
Mexican	5	6	12	16	1
White	34	115	115	32	3
Other	0	5	2	2	0

### 6.5.1 Cross-Classifying Three Categorical Variables

Suppose we are interested in `Smoke100` and its relationship to `PhysActive` and `SleepTrouble`.

```
xtabs(~ Smoke100 + PhysActive + SleepTrouble, data = nh_adults)
```

```
, , SleepTrouble = No
```

PhysActive		
Smoke100	No	Yes
No	99	135
Yes	62	66

```
, , SleepTrouble = Yes
```

PhysActive		
Smoke100	No	Yes
No	26	29
Yes	38	45

We can also build a `flat` version of this table, as follows:

```
ftable(Smoke100 ~ PhysActive + SleepTrouble, data = nh_adults)
```

	Smoke100		
PhysActive	SleepTrouble		
No	No	Yes	99 62
Yes	No	Yes	135 66
	26	38	29 45

And we can do this with `dplyr` functions, as well, for example...

```
nh_adults %>%
  select(Smoke100, PhysActive, SleepTrouble) %>%
  table()
```

```
, , SleepTrouble = No
```

PhysActive		
Smoke100	No	Yes
No	99	135
Yes	62	66

```
, , SleepTrouble = Yes
```

PhysActive		
Smoke100	No	Yes

No	26	29
Yes	38	45

## 6.6 Constructing Tables Well

The prolific Howard Wainer is responsible for many interesting books on visualization and related issues, including Wainer (2005) and Wainer (2013). These rules come from Chapter 10 of Wainer (1997).

1. Order the rows and columns in a way that makes sense.
2. Round, a lot!
3. ALL is different and important

### 6.6.1 Alabama First!

Which of these Tables is more useful to you?

2013 Percent of Students in grades 9-12 who are obese

State	% Obese	95% CI	Sample Size
Alabama	17.1	(14.6 - 19.9)	1,499
Alaska	12.4	(10.5-14.6)	1,167
Arizona	10.7	(8.3-13.6)	1,520
Arkansas	17.8	(15.7-20.1)	1,470
Connecticut	12.3	(10.2-14.7)	2,270
Delaware	14.2	(12.9-15.6)	2,475
Florida	11.6	(10.5-12.8)	5,491
...			
Wisconsin	11.6	(9.7-13.9)	2,771
Wyoming	10.7	(9.4-12.2)	2,910

or ...

State	% Obese	95% CI	Sample Size
Kentucky	18.0	(15.7 - 20.6)	1,537
Arkansas	17.8	(15.7 - 20.1)	1,470
Alabama	17.1	(14.6 - 19.9)	1,499
Tennessee	16.9	(15.1 - 18.8)	1,831
Texas	15.7	(13.9 - 17.6)	3,039
...			
Massachusetts	10.2	(8.5 - 12.1)	2,547
Idaho	9.6	(8.2 - 11.1)	1,841
Montana	9.4	(8.4 - 10.5)	4,679
New Jersey	8.7	(6.8 - 11.2)	1,644
Utah	6.4	(4.8 - 8.5)	2,136

It is a rare event when Alabama first is the best choice.

### 6.6.2 Order rows and columns sensibly

- Alabama First!
  - Size places - put the largest first. We often look most carefully at the top.
- Order time from the past to the future to help the viewer.
- If there is a clear predictor-outcome relationship, put the predictors in the rows and the outcomes in the columns.

### 6.6.3 Round - a lot!

- Humans cannot understand more than two digits very easily.
- We almost never care about accuracy of more than two digits.
- We can almost never justify more than two digits of accuracy statistically.
- It's also helpful to remember that we are almost invariably publishing progress to date, rather than a truly final answer.

Suppose, for instance, we report a correlation coefficient of 0.25. How many observations do you think you would need to justify such a choice?

- To report 0.25 meaningfully, we want to be sure that the second digit isn't 4 or 6.
- That requires a standard error less than 0.005
- The *standard error* of any statistic is proportional to 1 over the square root of the sample size,  $n$ .

So  $\frac{1}{\sqrt{n}} \sim 0.005$ , but that means  $\sqrt{n} = \frac{1}{0.005} = 200$ . If  $\sqrt{n} = 200$ , then  $n = (200)^2 = 40,000$ .

Do we usually have 40,000 observations?

### 6.6.4 ALL is different and important

Summaries of rows and columns provide a measure of what is typical or usual. Sometimes a sum is helpful, at other times, consider presenting a median or other summary. The ALL category, as Wainer (1997) suggests, should be both visually different from the individual entries and set spatially apart.

On the whole, it's *far* easier to fall into a good graph in R (at least if you have some ggplot2 skills) than to produce a good table.

# Chapter 7

## The National Youth Fitness Survey (nyfs1)

The `nyfs1.csv` data file comes from the 2012 National Youth Fitness Survey.

The NHANES National Youth Fitness Survey (NNYFS) was conducted in 2012 to collect data on physical activity and fitness levels in order to provide an evaluation of the health and fitness of children in the U.S. ages 3 to 15. The NNYFS collected data on physical activity and fitness levels of our youth through interviews and fitness tests.

In the `nyfs1.csv` data file, I'm only providing a tiny portion of the available information. More on the NNYFS (including information I'm not using) is available at the links below.

- Demographic Information including a complete description of all available variables.
- Body Measures, part of the general Examination data with complete variable descriptions

What I did was merge a few elements from the available demographic information with some elements from the body measures data, reformulated and simplified some variables, and restricted the sample to kids who had a complete set of body measure examinations.

### 7.1 Looking over the Data Set

To start with, I'll take a look at the `nyfs1` data. One approach is to simply get the size of the set and the names of the available data elements.

```
## first, we'll import the data into the nyfs1 data frame
nyfs1 <- read.csv("data/nyfs1.csv")

## next we'll turn that data frame into a more useful tibble
nyfs1 <-tbl_df(nyfs1)

## size of the data frame
dim(nyfs1)
```

```
[1] 1416    7
```

There are 1416 rows (subjects) and 7 columns (variables), by which I mean that there are 1416 kids in the `nyfs1` data frame, and we have 7 pieces of information on each subject.

So, what do we have, exactly?

```
nyfs1 # this is a tibble, has some nice features in a print-out like this
```

```
# A tibble: 1,416 x 7
  subject.id   sex age.exam   bmi      bmi.cat waist.circ
  <int> <fctr>    <int> <dbl>     <fctr>    <dbl>
1    71918 Female      8  22.3      4 Obese     71.9
2    71919 Female     14  19.8  2 Normal weight 79.4
3    71921 Male       3  15.2  2 Normal weight 46.8
4    71922 Male      12  25.9      4 Obese     90.0
5    71923 Male      12  22.5      3 Overweight 72.3
6    71924 Female     8  14.4  2 Normal weight 56.1
7    71925 Male       7  15.9  2 Normal weight 54.5
8    71926 Male      12  17.0  2 Normal weight 59.7
9    71927 Male       3  15.8  2 Normal weight 49.9
10   71928 Female     9  16.0  2 Normal weight 59.9
# ... with 1,406 more rows, and 1 more variables: triceps.skinfold <dbl>
```

Tibbles are a modern reimagining of the main way in which people have stored data in R, called a data frame. Tibbles were developed to keep what time has proven to be effective, and throwing out what is not. We can obtain the structure of the tibble from the `str` function.

```
str(nyfs1)
```

```
Classes 'tbl_df', 'tbl' and 'data.frame': 1416 obs. of 7 variables:
 $ subject.id : int 71918 71919 71921 71922 71923 71924 71925 71926 71927 71928 ...
 $ sex        : Factor w/ 2 levels "Female","Male": 1 1 2 2 2 1 2 2 2 1 ...
 $ age.exam   : int 8 14 3 12 12 8 7 8 3 9 ...
 $ bmi        : num 22.3 19.8 15.2 25.9 22.5 14.4 15.9 17 15.8 16 ...
 $ bmi.cat    : Factor w/ 4 levels "1 Underweight",...: 4 2 2 4 3 2 2 2 2 2 ...
 $ waist.circ: num 71.9 79.4 46.8 90 72.3 56.1 54.5 59.7 49.9 59.9 ...
 $ triceps.skinfold: num 19.9 15 8.6 22.8 20.5 12.9 6.9 8.8 10.8 13.2 ...
```

### 7.1.1 subject.id

The first variable, `subject.id` is listed by R as an `int` variable, for integer, which means it consists of whole numbers. However, the information provided by this variable is minimal. This is just an identifying code attributable to a given subject of the survey. This is *nominal* data, which will be of little interest down the line. On some occasions, as in this case, the ID numbers are sequential, in the sense that subject 71919 was included in the data base after subject 71918, but this fact isn't particularly interesting here, because the protocol remained unchanged throughout the study.

### 7.1.2 sex

The second variable, `sex` is listed as a factor (R uses `factor` to refer to categorical, especially non-numeric information) with two levels, *Female* and *Male*. You'll note that what is stored in the structure is a series of 1 (referring to the first level - Female) and 2 (Male) values. If we want to know how many people fall in each category, we can build a little table.

```
dplyr::select(nyfs1, sex) %>%
  table()
```

```
.
Female   Male
707     709
```

```
dplyr::select(nyfs1, sex) %>%
  table() %>%
  addmargins() ## add marginal totals

.
Female   Male   Sum
 707     709   1416

dplyr::select(nyfs1, sex) %>%
  table() %>%
  prop.table() ## look at the proportions instead
```

```
.
Female   Male
 0.499  0.501
```

Obviously, we don't actually need more than a couple of decimal places for any real purpose.

### 7.1.3 age.exam

The third variable, `age.exam` is the age of the child at the time of the examination, measured in years. Note that age is a continuous concept, but the measure used here (number of full years alive) is a common discrete approach to measurement. Age, of course, has a meaningful zero point, so this can be thought of as a ratio variable; a child who is 6 is half as old as one who is 12. We can get a table of the observed values.

```
dplyr::select(nyfs1, age.exam) %>%
  table() %>%
  addmargins()
```

```
.
      3    4    5    6    7    8    9    10   11   12   13   14   15   16   Sum
 97  111  119  129  123  120   90   109   102   108   113   104   85    6  1416
```

Note that some of the children apparently turned 16 between the time they were initially screened (when they were required to be between 3 and 15 years of age) and the time of the examination. The `sum` listed here is just the total count of all subjects. Since this is a meaningful quantitative variable, we may be interested in a more descriptive summary.

```
dplyr::select(nyfs1, age.exam) %>%
  summary()
```

```
age.exam
Min.    : 3.00
1st Qu.: 6.00
Median  : 9.00
Mean    : 8.86
3rd Qu.:12.00
Max.    :16.00
```

These six numbers provide a nice, if incomplete, look at the ages.

- `Min.` = the minimum, or youngest age at the examination was 3 years old.
- `1st Qu.` = the first quartile (25th percentile) of the ages was 6. This means that 25 percent of the subjects were age 6 or less.
- `Median` = the second quartile (50th percentile) of the ages was 9. This is often used to describe the center of the data. Half of the subjects were age 9 or less.
- `3rd Qu.` = the third quartile (75th percentile) of the ages was 12

- Max. = the maximum, or oldest age at the examination was 16 years.

### 7.1.4 bmi

The fourth variable, `bmi`, is the body-mass index of the child. The BMI is a person's weight in kilograms divided by his or her height in meters squared. Symbolically,  $\text{BMI} = \text{weight in kg} / (\text{height in m})^2$ . This is a continuous concept, measured to as many decimal places as you like, and it has a meaningful zero point, so it's a ratio variable.

```
dplyr::select(nyfs1, bmi) %>%
  summary()
```

```
bmi
Min.   :11.9
1st Qu.:15.8
Median :17.7
Mean   :18.8
3rd Qu.:20.9
Max.   :38.8
```

Why would a table of these BMI values not be a great idea, for these data? A hint is that R represents this variable as `num` or numeric in its depiction of the data structure, and this implies that R has some decimal values stored.

```
dplyr::select(nyfs1, bmi) %>%
  table()
```

11.9	12.6	12.7	12.9	13	13.1	13.2	13.3	13.4	13.5	13.6	13.7	13.8	13.9	14
1	1	1	1	2	2	1	1	3	4	5	4	5	11	7
14.1	14.2	14.3	14.4	14.5	14.6	14.7	14.8	14.9	15	15.1	15.2	15.3	15.4	15.5
12	9	11	11	11	9	17	20	23	13	14	18	27	24	32
15.6	15.7	15.8	15.9	16	16.1	16.2	16.3	16.4	16.5	16.6	16.7	16.8	16.9	17
18	20	21	30	27	15	18	30	12	25	20	22	13	21	23
17.1	17.2	17.3	17.4	17.5	17.6	17.7	17.8	17.9	18	18.1	18.2	18.3	18.4	18.5
14	20	14	10	19	13	17	18	14	17	13	10	9	8	15
18.6	18.7	18.8	18.9	19	19.1	19.2	19.3	19.4	19.5	19.6	19.7	19.8	19.9	20
10	17	10	11	4	13	15	12	8	25	6	6	16	8	13
20.1	20.2	20.3	20.4	20.5	20.6	20.7	20.8	20.9	21	21.1	21.2	21.3	21.4	21.5
9	7	12	7	3	9	5	6	11	7	5	6	8	9	8
21.6	21.7	21.8	21.9	22	22.1	22.2	22.3	22.4	22.5	22.6	22.7	22.8	22.9	23
6	7	16	6	13	7	7	8	6	4	4	5	2	10	7
23.1	23.2	23.3	23.4	23.5	23.6	23.7	23.8	23.9	24	24.1	24.2	24.3	24.4	24.5
3	8	3	5	4	3	2	4	4	5	1	4	3	5	5
24.6	24.7	24.8	24.9	25	25.1	25.2	25.3	25.4	25.5	25.6	25.7	25.8	25.9	26
4	3	6	4	3	2	4	2	3	3	4	5	3	3	2
26.1	26.2	26.3	26.4	26.5	26.6	26.7	26.8	27	27.2	27.3	27.4	27.5	27.6	27.7
1	4	2	1	2	1	2	1	2	1	2	2	1	2	1
27.9	28.1	28.2	28.4	28.5	28.6	28.7	28.8	28.9	29	29.2	29.5	29.7	29.8	30.1
2	2	2	1	1	2	2	1	3	1	3	1	2	3	2
30.2	30.4	30.5	30.7	30.8	30.9	31.1	31.3	31.4	31.5	31.7	31.8	32	32.2	32.4
4	1	2	1	1	1	1	1	2	1	1	2	2	1	1
32.6	32.9	33.2	33.5	34	34.4	34.6	34.7	35.9	37	38.8				
1	1	1	1	1	1	1	1	1	1	1	1			

### 7.1.5 bmi.cat

Our next variable, `bmi.cat`, is a four-category ordinal variable, which divides the sample according to BMI into four groups. The BMI categories use sex-specific 2000 BMI-for-age (in months) growth charts prepared by the Centers for Disease Control for the US. We can get the breakdown from a table of the variable's values.

```
dplyr::select(nyfs1, bmi.cat) %>%
  table() %>%
  addmargins()
```

1 Underweight	2 Normal weight	3 Overweight	4 Obese
42	926	237	211
Sum			
1416			

In terms of percentiles by age and sex from the growth charts, the meanings of the categories are:

- Underweight ( $\text{BMI} < 5\text{th percentile}$ )
- Normal weight ( $\text{BMI } 5\text{th to } < 85\text{th percentile}$ )
- Overweight ( $\text{BMI } 85\text{th to } < 95\text{th percentile}$ )
- Obese ( $\text{BMI} \geq 95\text{th percentile}$ )

Note how I've used labels in the `bmi.cat` variable that include a number at the start so that the table results are sorted in a rational way. R sorts tables alphabetically, in general.

### 7.1.6 waist.circ

The sixth variable is `waist.circ`, which is the circumference of the child's waist, in centimeters. Again, this is a numeric variable, so perhaps we'll stick to the simple summary, rather than obtaining a table of observed values.

```
dplyr::select(nyfs1, waist.circ) %>%
  summary()
```

```
waist.circ
Min.    : 42.5
1st Qu.: 55.0
Median  : 63.0
Mean    : 65.3
3rd Qu.: 72.9
Max.    :112.4
```

### 7.1.7 triceps.skinfold

The seventh and final variable is `triceps.skinfold`, which is measured in millimeters. This is one of several common locations used for the assessment of body fat using skinfold calipers, and is a frequent part of growth assessments in children. Again, this is a numeric variable according to R.

```
dplyr::select(nyfs1, triceps.skinfold) %>%
  summary()
```

```
triceps.skinfold
Min.    : 4.0
1st Qu.: 9.0
Median  :11.8
```

```
Mean    :13.4
3rd Qu.:16.6
Max.   :38.2
```

## 7.2 Summarizing the Data Set

The **summary** function can be applied to the whole tibble. For numerical and integer variables, this function produces the five number summary, plus the mean. For categorical (factor) variables, it lists the count for each category.

```
summary(nyfs1)
```

```
subject.id      sex       age.exam      bmi
Min.    :71918  Female:707  Min.    : 3.00  Min.    :11.9
1st Qu.:72313  Male   :709   1st Qu.: 6.00  1st Qu.:15.8
Median  :72698                           Median : 9.00  Median :17.7
Mean    :72703                           Mean   : 8.86  Mean   :18.8
3rd Qu.:73096                           3rd Qu.:12.00 3rd Qu.:20.9
Max.    :73492                           Max.   :16.00  Max.   :38.8
bmi.cat        waist.circ  triceps.skinfold
1 Underweight  : 42   Min.    :42.5   Min.    : 4.0
2 Normal weight:926  1st Qu.:55.0   1st Qu.: 9.0
3 Overweight   :237   Median  :63.0   Median :11.8
4 Obese        :211   Mean    :65.3   Mean   :13.4
                           3rd Qu.:72.9   3rd Qu.:16.6
                           Max.   :112.4  Max.   :38.2
```

### 7.2.1 The Five Number Summary, Quantiles and IQR

The **five number summary** is most famous when used to form a box plot - it's the minimum, 25th percentile, median, 75th percentile and maximum. Our usual **summary** adds the mean.

```
nyfs1 %>%
  select(bmi) %>%
  summary()
```

```
bmi
Min.    :11.9
1st Qu.:15.8
Median  :17.7
Mean    :18.8
3rd Qu.:20.9
Max.   :38.8
```

As an alternative, we can use the \$ notation to indicate the variable we wish to study inside a data set, and we can use the **fivenum** function to get the five numbers used in developing a box plot.

```
fivenum(nyfs1$bmi)
```

```
[1] 11.9 15.8 17.7 20.9 38.8
```

- As mentioned in 5.3.1, the **inter-quartile range**, or IQR, is sometimes used as a competitor for the standard deviation. It's the difference between the 75th percentile and the 25th percentile. The 25th percentile, median, and 75th percentile are referred to as the quartiles of the data set, because, together, they split the data into quarters.

```
IQR(nyfs1$bmi)
```

```
[1] 5.1
```

We can obtain **quantiles** (percentiles) as we like - here, I'm asking for the 1st and 99th

```
quantile(nyfs1$bmi, probs=c(0.01, 0.99))
```

```
1%   99%
13.5 32.0
```

## 7.3 Additional Summaries from favstats

If we're focusing on a single variable, the **favstats** function in the **mosaic** package can be very helpful. Rather than calling up the entire **mosaic** library here, I'll just specify the function within the library.

```
mosaic::favstats(nyfs1$bmi)
```

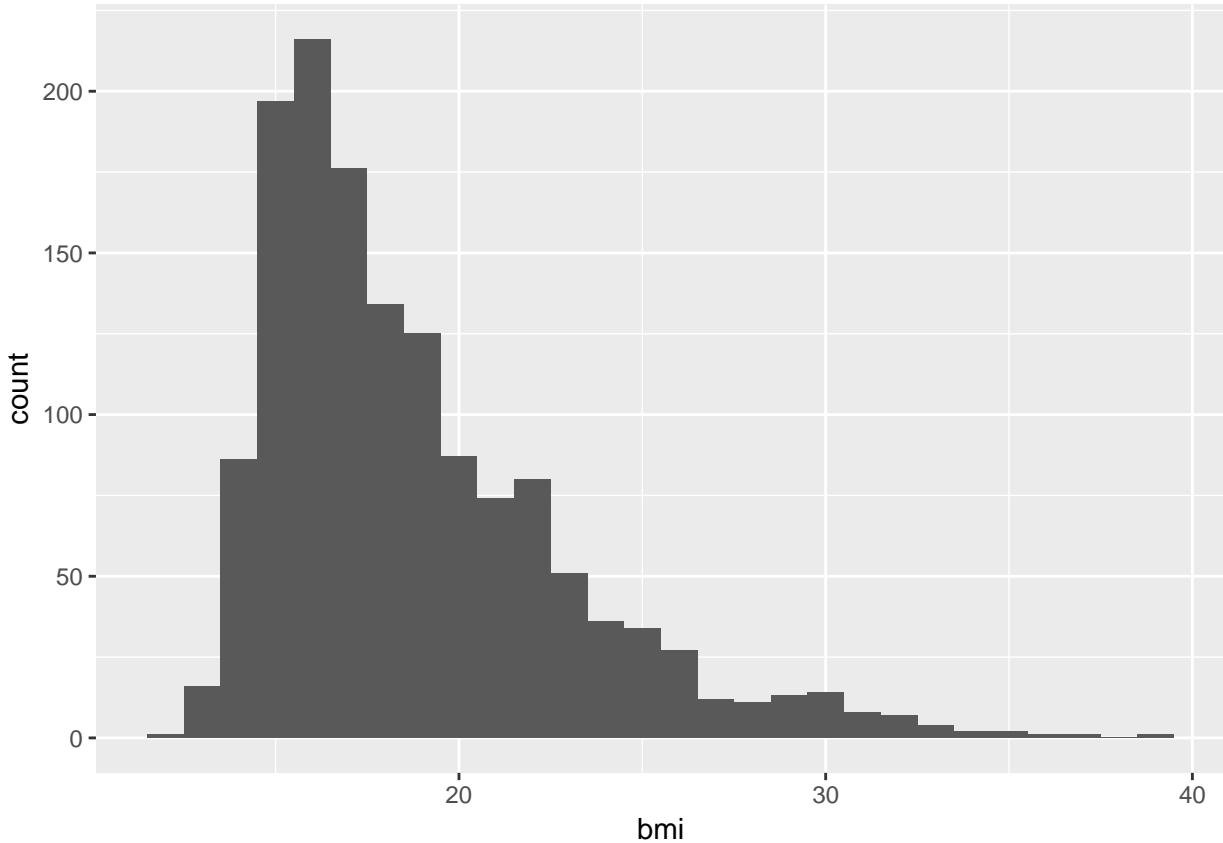
```
min   Q1 median   Q3  max mean   sd      n missing
11.9 15.8   17.7 20.9 38.8 18.8 4.08 1416       0
```

This adds three useful results to the base summary - the standard deviation, the sample size and the number of missing observations.

## 7.4 The Histogram

As we saw in 3, obtaining a basic **histogram** of, for example, the BMIs in the **nyfs1** data is pretty straightforward.

```
ggplot(data = nyfs1, aes(x = bmi)) +
  geom_histogram(binwidth = 1)
```



#### 7.4.1 Freedman-Diaconis Rule to select bin width

If we like, we can suggest a particular number of cells for the histogram, instead of accepting the defaults. In this case, we have  $n = 1416$  observations. The **Freedman-Diaconis rule** can be helpful here. That rule suggests that we set the bin-width to

$$h = \frac{2 * IQR}{n^{1/3}}$$

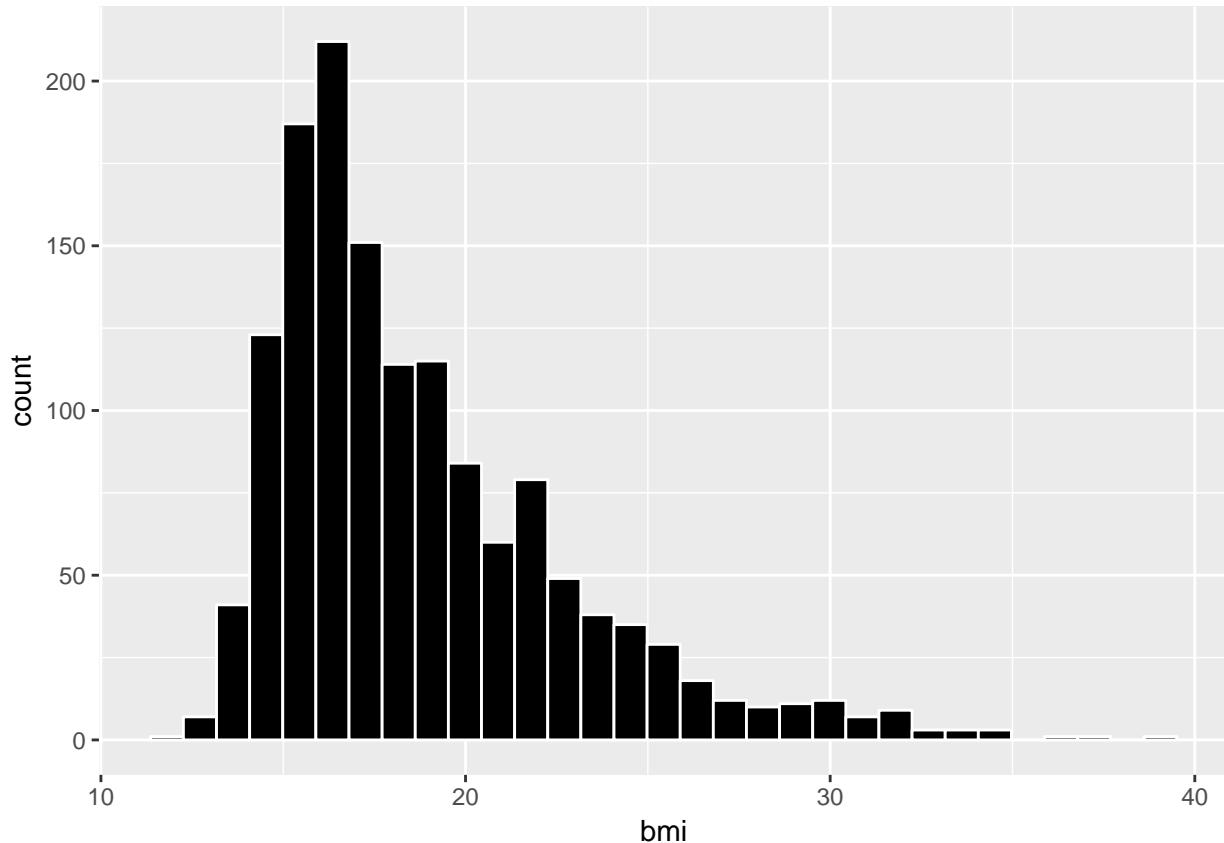
so that the number of bins is equal to the range of the data set (maximum - minimum) divided by  $h$ .

For the `bmi` data in the `nyfs1` tibble, we have

- IQR of 5.1,  $n = 1416$  and range = 26.9
- Thus, by the Freedman-Diaconis rule, the optimal binwidth  $h$  is 0.908, or, realistically, 1.
- And so the number of bins would be 29.615, or, realistically 30.

Here, we'll draw the graph again, using the Freedman-Diaconis rule to identify the number of bins, and also play around a bit with the fill and color of the bars.

```
bw <- 2 * IQR(nyfs1$bmi) / length(nyfs1$bmi)^(1/3)
ggplot(data = nyfs1, aes(x = bmi)) +
  geom_histogram(binwidth=bw, color = "white", fill = "black")
```

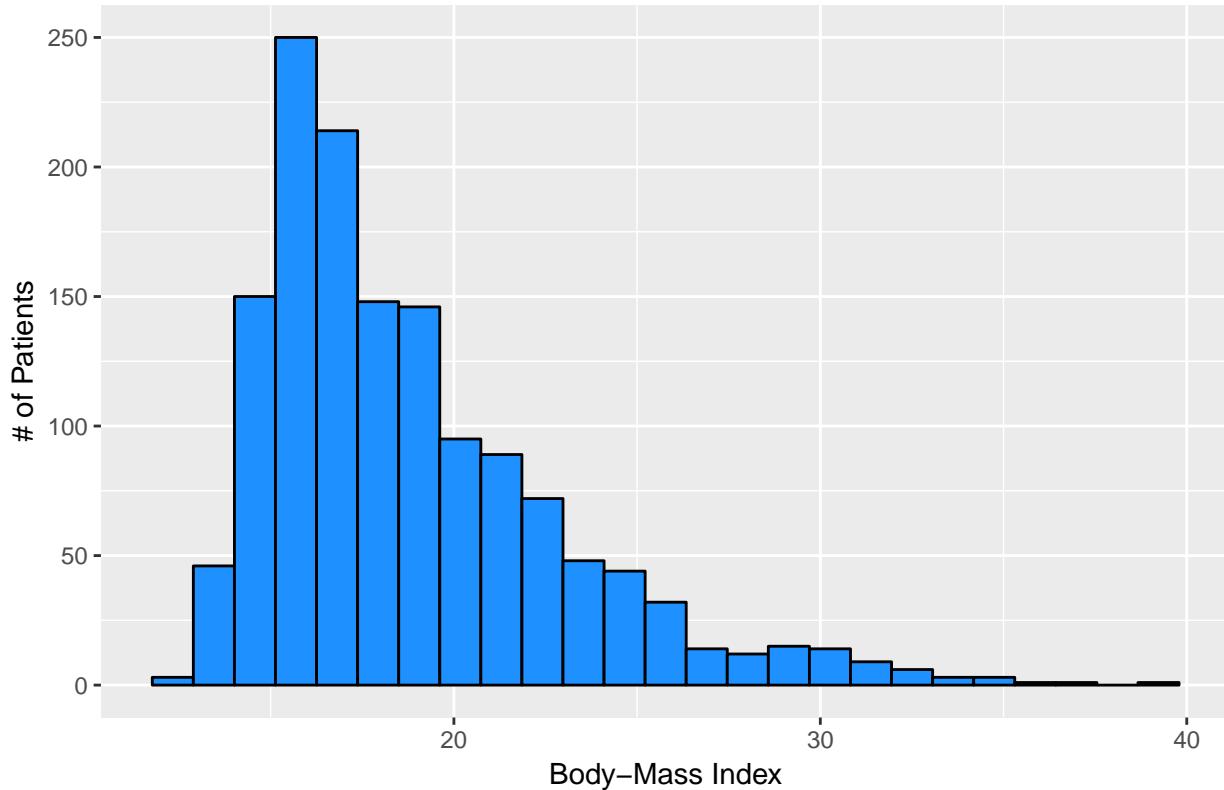


This is a nice start, but it is by no means a finished graph.

Let's improve the axis labels, add a title, and fill in the bars with a distinctive blue and use a black outline around each bar. I'll just use 25 bars, because I like how that looks in this case, and optimizing the number of bins is rarely important.

```
ggplot(data = nyfs1, aes(x = bmi)) +
  geom_histogram(bins=25, color = "black", fill = "dodgerblue") +
  labs(title = "Histogram of Body-Mass Index Results in the nyfs1 data",
       x = "Body-Mass Index", y = "# of Patients")
```

### Histogram of Body–Mass Index Results in the nyfs1 data



## 7.5 A Note on Colors

The simplest way to specify a color is with its name, enclosed in parentheses. My favorite list of R colors is <http://www.stat.columbia.edu/~tzhang/files/Rcolor.pdf>. In a pinch, you can find it by googling **Colors in R**. You can also type `colors()` in the R console to obtain a list of the names of the same 657 colors.

When using colors to make comparisons, you may be interested in using a scale that has some nice properties. I suggest the `viridis` package to help with this work. The `viridis` package vignette describes four color scales (`viridis`, `magma`, `plasma` and `inferno`) that are designed to be colorful, robust to colorblindness and gray scale printing, and perceptually uniform, which means (as the package authors describe it) that values close to each other have similar-appearing colors and values far away from each other have more different-appearing colors, consistently across the range of values.

## 7.6 The Stem-and-Leaf

We might consider a **stem-and-leaf display** (a John Tukey invention) to show the actual data values while retaining the shape of a histogram. The `scale` parameter can help expand the size of the diagram, so you can see more of the values. Stem and leaf displays are usually used for relatively small samples, perhaps with 10-200 observations, so we'll first take a sample of 150 of the BMI values from the complete set gathered in the `nyfs1` tibble.

```
set.seed(431) # set a seed for the random sampling so we can replicate the results
```

```
sampleA <- sample_n(nyfs1, 150, replace = FALSE) # draw a sample of 150 unique rows from nyfs1
stem(sampleA$bmi) # build a stem-and-leaf for those 150 sampled BMI values
```

The decimal point is at the |

```
13 | 129
14 | 001224455566778889
15 | 02344455567789999
16 | 0000112233345667779
17 | 001225556677789
18 | 0111346677888899
19 | 111224555578889
20 | 0113334456899
21 | 014568
22 | 11349
23 | 012479
24 | 478
25 | 05669
26 | 03
27 | 05
28 |
29 |
30 | 27
31 |
32 | 4
33 |
34 | 67
```

We can see that the minimum BMI value in this small sample is 13.1 and the maximum BMI value is 34.7.

Here's a summary of all variables for these 150 observations.

```
summary(sampleA)
```

	subject.id	sex	age.exam	bmi
Min.	:71935	Female:68	Min. : 3.00	Min. :13.1
1st Qu.	:72302	Male :82	1st Qu.: 6.00	1st Qu.:15.9
Median	:72688		Median :10.00	Median :18.1
Mean	:72679		Mean : 9.45	Mean :19.0
3rd Qu.	:73080		3rd Qu.:13.00	3rd Qu.:20.6
Max.	:73490		Max. :15.00	Max. :34.7
		bmi.cat	waist.circ	triceps.skinfold
1	Underweight	: 4	Min. : 45.6	Min. : 5.6
2	Normal weight	:103	1st Qu.: 55.4	1st Qu.: 9.2
3	Overweight	: 21	Median : 64.7	Median :12.2
4	Obese	: 22	Mean : 66.5	Mean :13.6
			3rd Qu.: 72.8	3rd Qu.:16.6
			Max. :108.4	Max. :34.8

If we really wanted to, we could obtain a stem-and-leaf of all of the BMI values in the entire `nyfs1` data. The `scale` parameter lets us see some more of the values.

```
stem(nyfs1$bmi, scale = 2)
```

The decimal point is at the |

```

11 | 9
12 | 679
13 | 001123444555666667778888999999999999
14 | 00000001111111111122222222333333333444444445555555556666666+50
15 | 000000000000011111111111222222222223333333333333333333333333+137
16 | 00000000000000000000000000000000001111111111122222222222233333333333333+123
17 | 000000000000000000000000000000000011111111111222222222222333333333333333+82
18 | 00000000000000000000000000000000001111111111122222222223333333344444445555555555555+40
19 | 000011111111111112222222222223333333333344444444555555555555555+33
20 | 0000000000000000111111111222222233333333333344444445556666666677777888+2
21 | 00000001111122222333333334444444455555556666667777788888888888
22 | 0000000000000001111111222222333333334444444555566667777788999999999
23 | 000000011122222223334444455556667788889999
24 | 000001222233344444555566677888889999
25 | 000112222334455566677777888999
26 | 0012222334556778
27 | 0023344566799
28 | 11224566778999
29 | 0222577888
30 | 112222455789
31 | 13445788
32 | 002469
33 | 25
34 | 0467
35 | 9
36 |
37 | 0
38 | 8

```

Note that some of the rows extend far beyond what is displayed in the data (as indicated by the + sign, followed by a count of the number of unshown data values.)

### 7.6.1 A Fancier Stem-and-Leaf Display

We can use the `stem.leaf` function in the `aplypack` package to obtain a fancier version of the stem-and-leaf plot, that identifies outlying values. Below, we display this new version for the random sample of 150 BMI observations we developed earlier.

```
aplypack::stem.leaf(sampleA$bmi)
```

```

1 | 2: represents 1.2
leaf unit: 0.1
n: 150
 3   13 | 129
 21  14 | 001224455566778889
 38  15 | 02344455567789999
 57  16 | 0000112233345667779
 72  17 | 001225556677789
(16) 18 | 0111346677888899
 62  19 | 111224555578889
 47  20 | 0113334456899
 34  21 | 014568

```

```

28    22 | 11349
23    23 | 012479
17    24 | 478
14    25 | 05669
 9    26 | 03
 7    27 | 05
HI: 30.2 30.7 32.4 34.6 34.7

```

We can also produce back-to-back stem and leaf plots to compare, for instance, body-mass index by sex.

```

samp.F <- filter(sampleA, sex=="Female")
samp.M <- filter(sampleA, sex=="Male")

aplypack::stem.leaf.backback(samp.F$bmi, samp.M$bmi)

```

```

-----
1 | 2: represents 1.2, leaf unit: 0.1
      samp.F$bmi      samp.M$bmi
-----
3          921| 13 |
16   9876654422100| 14 |55788           5
21       98444| 15 |023555677999  17
33   776653210000| 16 |1233479        24
(2)      91| 17 |0022555667778  37
33       9887410| 18 |113667889     (9)
26       9888555411| 19 |12257        36
16       9954310| 20 |133468        31
 9         0| 21 |14568        25
          | 22 |11349        20
 8         910| 23 |247        15
 5           8| 24 |47        12
 4         95| 25 |066        10
          | 26 |03          7
          | 27 |05          5
          | 28 |           1
-----
HI: 30.2 32.4          HI: 30.7 34.6 34.7
n:          68          82
-----
```

## 7.7 The Dot Plot to display a distribution

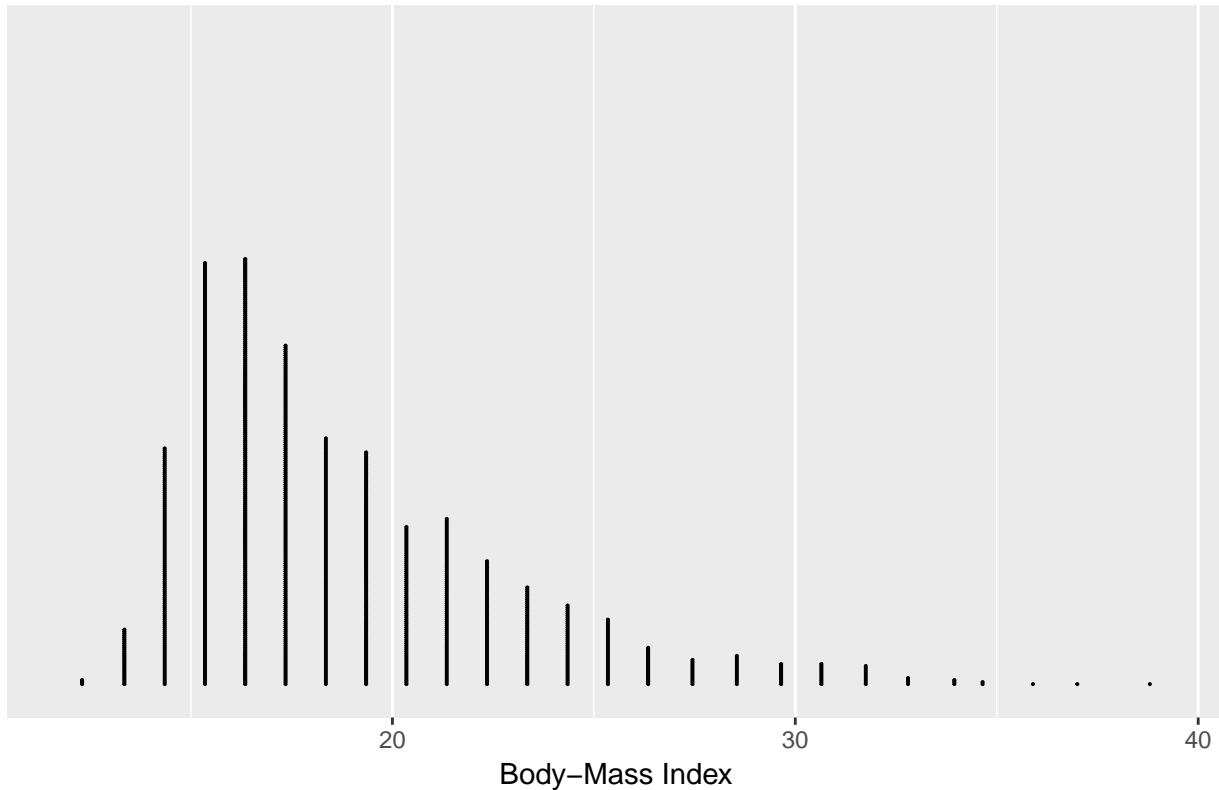
We can plot the distribution of a single continuous variable using the `dotplot` geom:

```

ggplot(data = nyfs1, aes(x = bmi)) +
  geom_dotplot(dotsize = 0.05, binwidth=1) +
  scale_y_continuous(NULL, breaks = NULL) + # hides y-axis since it is meaningless
  labs(title = "Dotplot of nyfs1 Body-Mass Index data",
       x = "Body-Mass Index")

```

### Dotplot of nyfs1 Body–Mass Index data

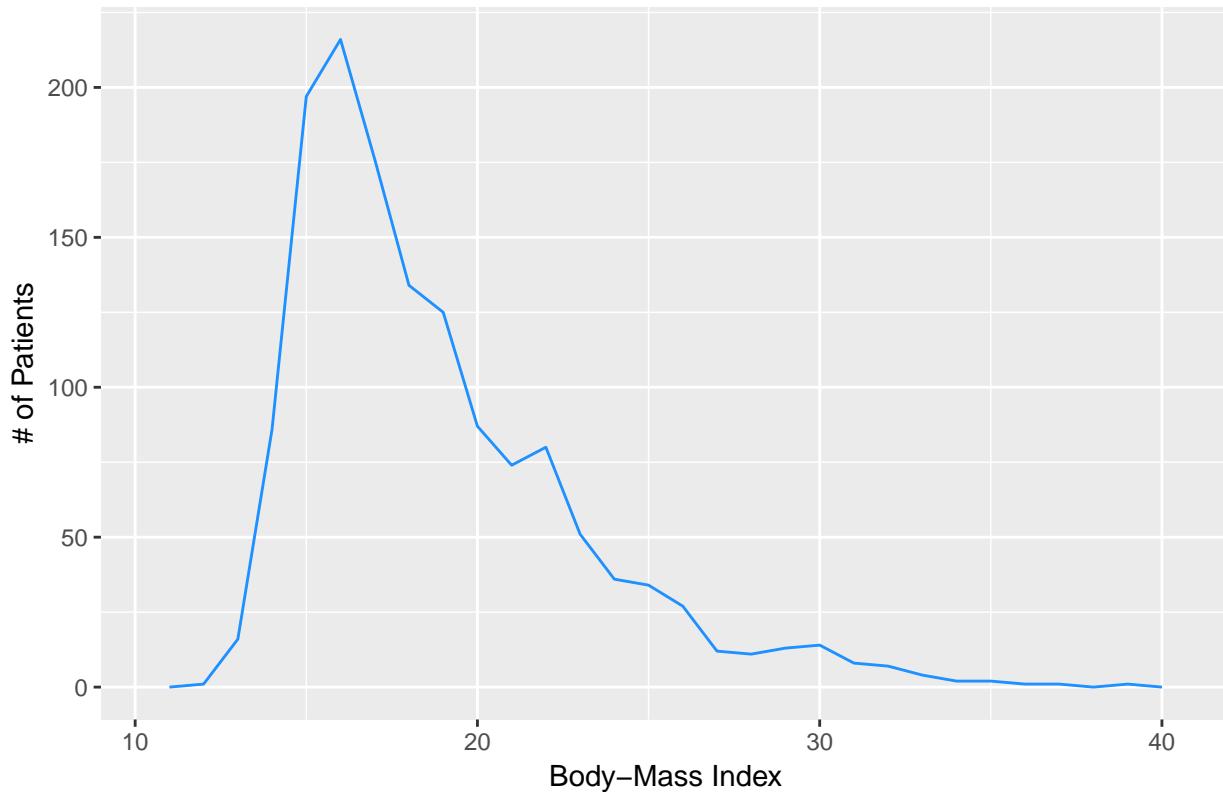


## 7.8 The Frequency Polygon

We can plot the distribution of a single continuous variable using the `freqpoly` geom:

```
ggplot(data = nyfs1, aes(x = bmi)) +  
  geom_freqpoly(binwidth = 1, color = "dodgerblue") +  
  labs(title = "Frequency Polygon of nyfs1 Body-Mass Index data",  
       x = "Body-Mass Index", y = "# of Patients")
```

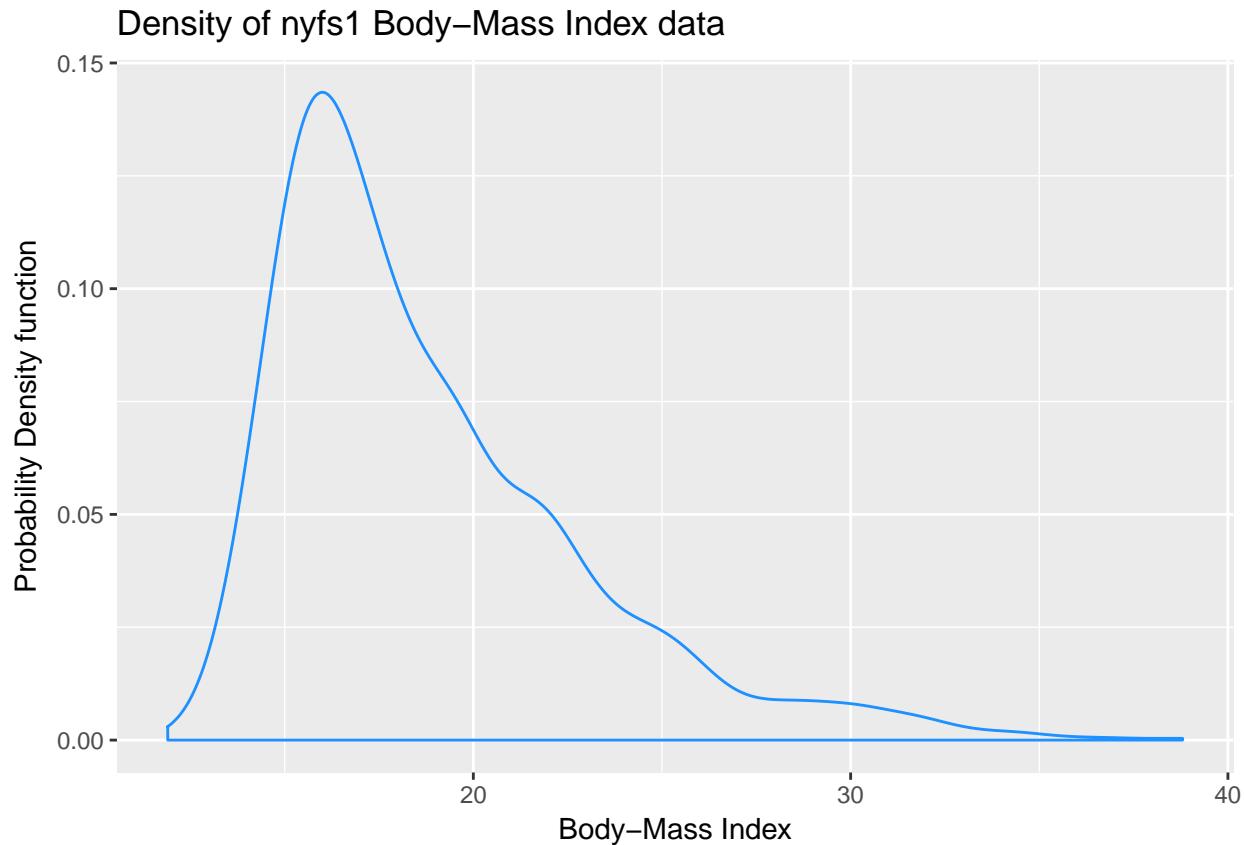
Frequency Polygon of nyfs1 Body–Mass Index data



## 7.9 Plotting the Probability Density Function

We can also produce a density function, which has the effect of smoothing out the bumps in a histogram or frequency polygon, while also changing what is plotted on the y-axis.

```
ggplot(data = nyfs1, aes(x = bmi)) +
  geom_density(kernel = "gaussian", color = "dodgerblue") +
  labs(title = "Density of nyfs1 Body–Mass Index data",
       x = "Body–Mass Index", y = "Probability Density function")
```



So, what's a density function?

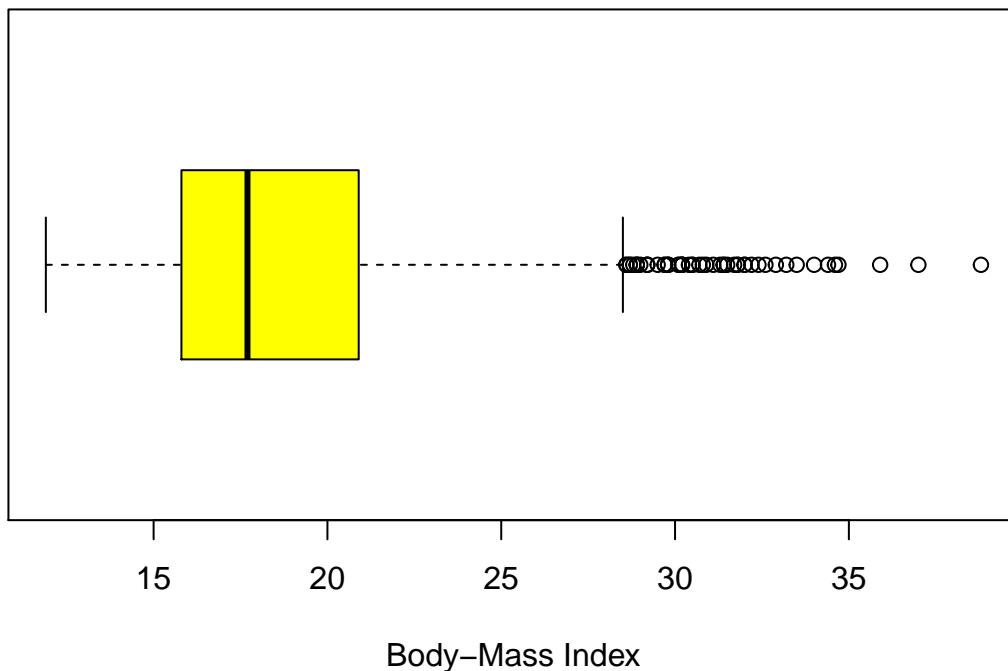
- A probability density function is a function of a continuous variable,  $x$ , that represents the probability of  $x$  falling within a given range. Specifically, the integral over the interval  $(a,b)$  of the density function gives the probability that the value of  $x$  is within  $(a,b)$ .
- If you're interested in exploring more on the notion of density functions for continuous (and discrete) random variables, some nice elementary material is available at Khan Academy.

## 7.10 The Boxplot

Sometimes, it's helpful to picture the five-number summary of the data in such a way as to get a general sense of the distribution. One approach is a **boxplot**, sometimes called a box-and-whisker plot.

```
boxplot(nyfs1$bmi, col="yellow", horizontal=T, xlab="Body–Mass Index",
       main="BMI for 1416 kids in the NYFS")
```

## BMI for 1416 kids in the NYFS



The boxplot is another John Tukey invention.

- R draws the box (here in yellow) so that its edges of the box fall at the 25<sup>th</sup> and 75<sup>th</sup> percentiles of the data, and the thick line inside the box falls at the median (50<sup>th</sup> percentile).
- The whiskers then extend out to the largest and smallest values that are not classified by the plot as candidate *outliers*.
- An outlier is an unusual point, far from the center of a distribution.
- Note that I've used the `horizontal` option to show this boxplot in this direction. Most comparison boxplots, as we'll see below, are oriented vertically.

The boxplot's **whiskers** that are drawn from the first and third quartiles (i.e. the 25<sup>th</sup> and 75<sup>th</sup> percentiles) out to the most extreme points in the data that do not meet the standard of "candidate outliers." An outlier is simply a point that is far away from the center of the data - which may be due to any number of reasons, and generally indicates a need for further investigation.

Most software, including R, uses a standard proposed by Tukey which describes a "candidate outlier" as any point above the **upper fence** or below the **lower fence**. The definitions of the fences are based on the inter-quartile range (IQR).

If  $IQR = 75\text{th} \text{ percentile} - 25\text{th} \text{ percentile}$ , then the upper fence is  $75\text{th} \text{ percentile} + 1.5 \times IQR$ , and the lower fence is  $25\text{th} \text{ percentile} - 1.5 \times IQR$ .

So for these BMI data,

- the upper fence is located at  $20.9 + 1.5(5.1) = 28.55$
- the lower fence is located at  $15.8 - 1.5(5.1) = 8.15$

In this case, we see no points identified as outliers in the low part of the distribution, but quite a few identified that way on the high side. This tends to identify about 5% of the data as a candidate outlier, *if* the data follow a Normal distribution.

- This plot is indicating clearly that there is some asymmetry (skew) in the data, specifically right skew.
- The standard R uses is to indicate as outliers any points that are more than 1.5 inter-quartile ranges away from the edges of the box.

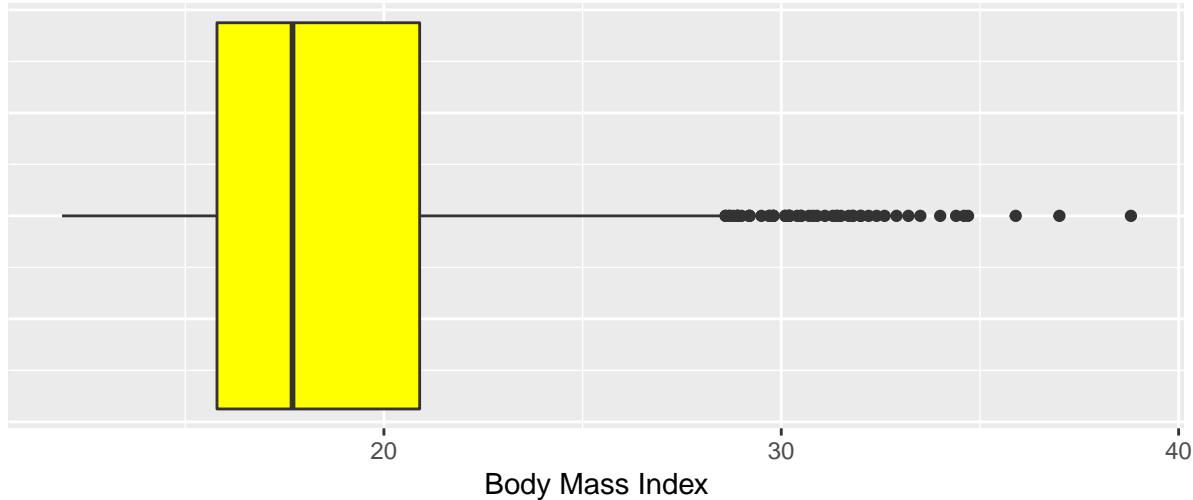
The horizontal orientation I've chosen here clarifies the relationship of direction of skew to the plot. A plot like this, with multiple outliers on the right side is indicative of a long right tail in the distribution, and hence, positive or right skew - with the mean being larger than the median. Other indications of skew include having one side of the box being substantially wider than the other, or one side of the whiskers being substantially longer than the other. More on skew later.

### 7.10.1 Drawing a Boxplot for One Variable in ggplot2

The `ggplot2` library easily handles comparison boxplots for multiple distributions, as we'll see in a moment. However, building a boxplot for a single distribution requires a little trickiness.

```
ggplot(nyfs1, aes(x = 1, y = bmi)) +
  geom_boxplot(fill = "yellow") +
  coord_flip() +
  labs(title = "Boxplot of BMI for 1416 kids in the NYFS",
       y = "Body Mass Index",
       x = "") +
  theme(axis.text.y = element_blank(),
        axis.ticks.y = element_blank())
```

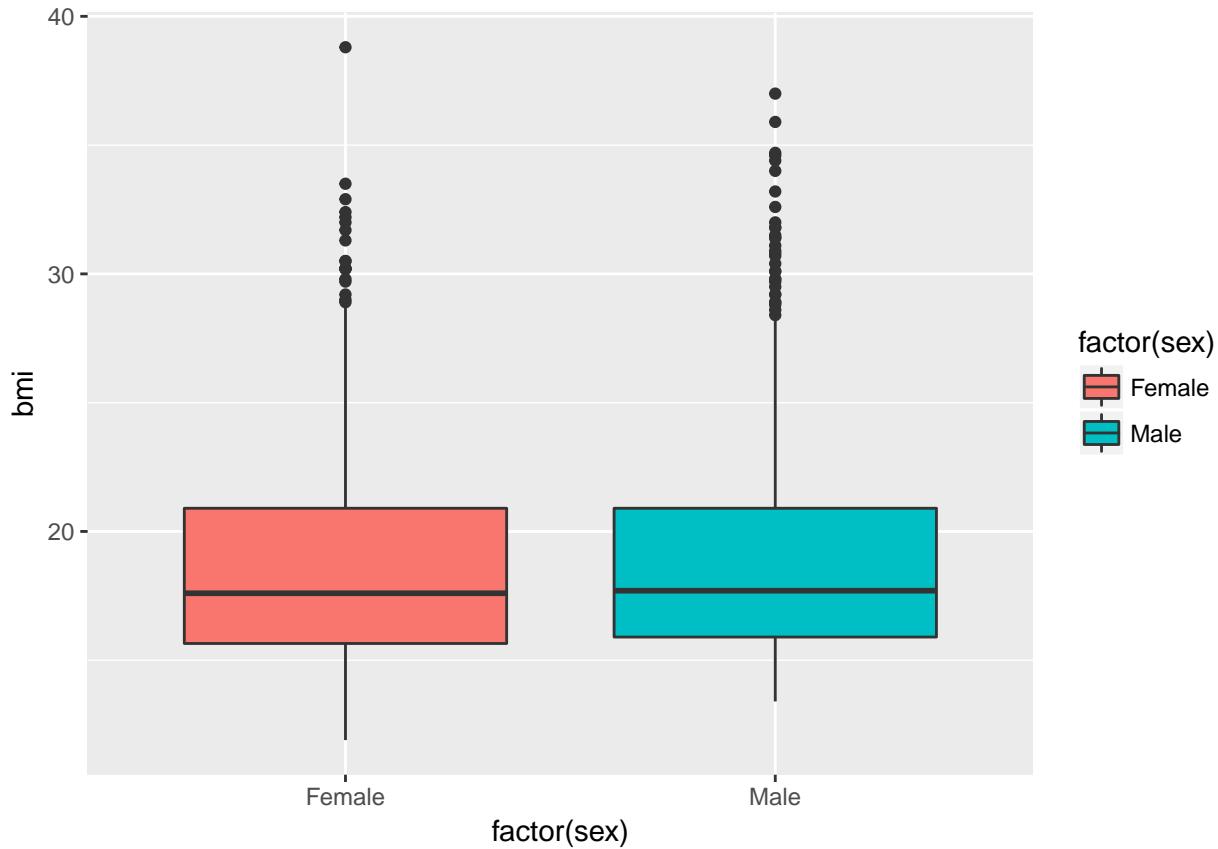
Boxplot of BMI for 1416 kids in the NYFS



## 7.11 A Simple Comparison Boxplot

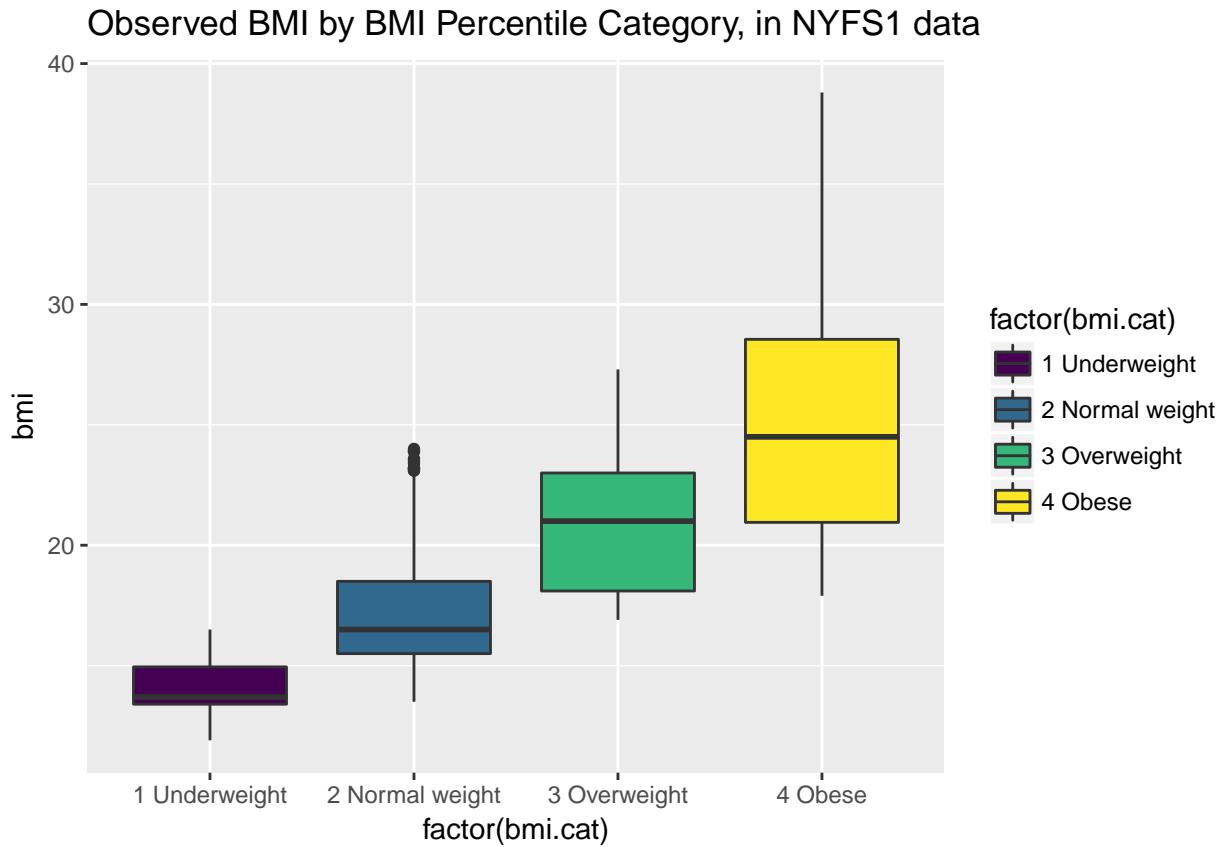
Boxplots are most often used for comparison. We can build boxplots using `ggplot2`, as well, and we'll discuss that in detail later. For now, here's a boxplot built to compare the `bmi` results by the child's sex.

```
ggplot(nyfs1, aes(x = factor(sex), y = bmi, fill=factor(sex))) +
  geom_boxplot()
```



Let's look at the comparison of observed BMI levels across the four categories in our `bmi.cat` variable, now making use of the `viridis` color scheme.

```
ggplot(nyfs1, aes(x = factor(bmi.cat), y = bmi, fill = factor(bmi.cat))) +
  geom_boxplot() +
  scale_fill_viridis(discrete=TRUE) +
  # above line uses viridis palette to identify color choices
  labs(title = "Observed BMI by BMI Percentile Category, in NYFS1 data")
```

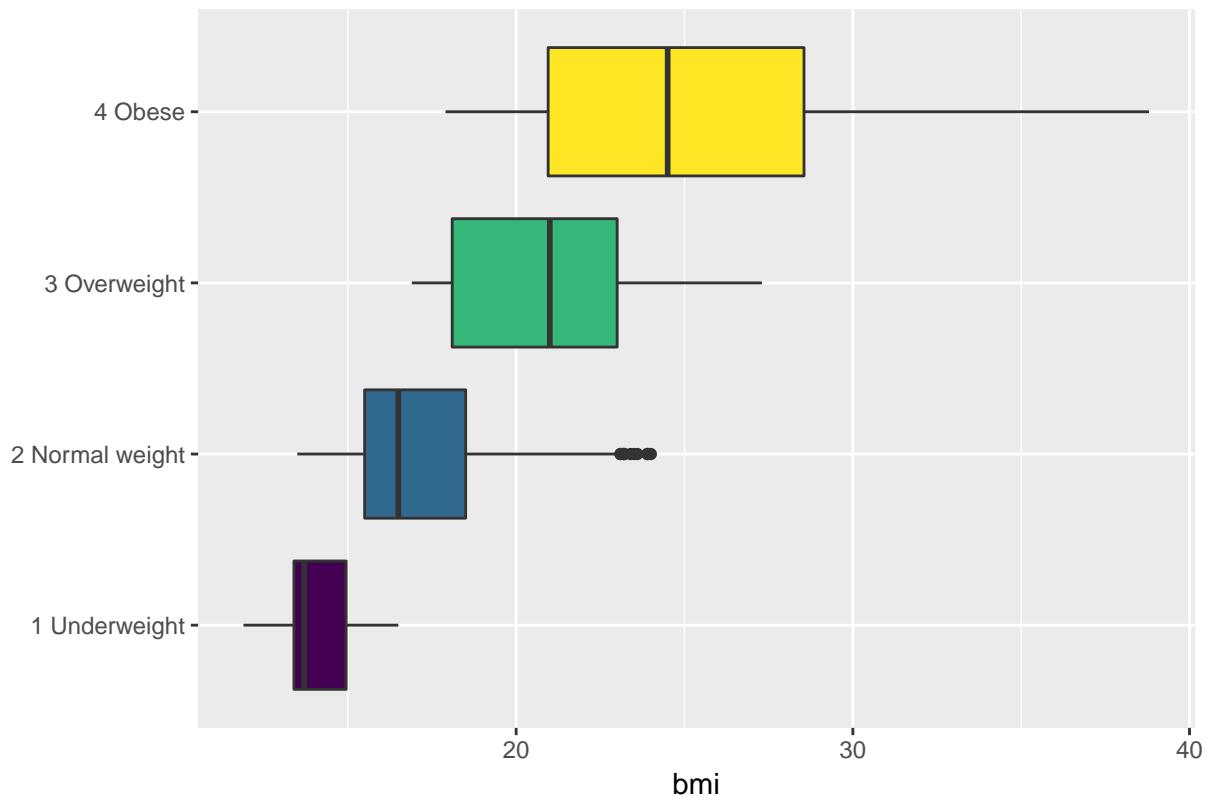


Note that the BMI categories incorporate additional information (in particular the age and sex of the child) beyond the observed BMI, and so the observed BMI levels overlap quite a bit across the four categories. As a graph, that's not bad, but what if we want to improve it further?

Let's turn the boxes in the horizontal direction, and get rid of the perhaps unnecessary `bmi.cat` labels.

```
ggplot(nyfs1, aes(x = factor(bmi.cat), y = bmi, fill = factor(bmi.cat))) +
  geom_boxplot() +
  scale_fill_viridis(discrete=TRUE) +
  coord_flip() +
  guides(fill=FALSE) +
  labs(title = "Observed BMI by BMI Percentile Category, in NYFS1 data", x = "")
```

Observed BMI by BMI Percentile Category, in NYFS1 data



## 7.12 Using `describe` in the `psych` library

For additional numerical summaries, one option would be to consider using the `describe` function from the `psych` library.

```
psych::describe(nyfs1$bmi)
```

```
vars     n  mean    sd median trimmed   mad   min   max range skew kurtosis
X1      1 1416 18.8  4.08    17.7   18.2 3.26 11.9 38.8 26.9 1.35     1.97
      se
X1 0.11
```

This package provides, in order, the following...

- `n` = the sample size
- `mean` = the sample mean
- `sd` = the sample standard deviation
- `median` = the median, or 50th percentile
- `trimmed` = mean of the middle 80% of the data
- `mad` = median absolute deviation
- `min` = minimum value in the sample
- `max` = maximum value in the sample
- `range` = max - min
- `skew` = skewness measure, described below (indicates degree of asymmetry)
- `kurtosis` = kurtosis measure, described below (indicates heaviness of tails, degree of outlier-proneness)
- `se` = standard error of the sample mean =  $sd / \sqrt{n}$ , useful in inference

### 7.12.1 The Trimmed Mean

The **trimmed mean** trim value in R indicates proportion of observations to be trimmed from each end of the outcome distribution before the mean is calculated. The **trimmed** value provided by the `psych::describe` package describes what this particular package calls a 20% trimmed mean (bottom and top 10% of BMIs are removed before taking the mean - it's the mean of the middle 80% of the data.) I might call that a 10% trimmed mean in some settings, but that's just me.

```
mean(nyfs1$bmi, trim=.1)
```

```
[1] 18.2
```

### 7.12.2 The Median Absolute Deviation

An alternative to the IQR that is fancier, and a bit more robust, is the **median absolute deviation**, which, in large sample sizes, for data that follow a Normal distribution, will be (in expectation) equal to the standard deviation. The MAD is the median of the absolute deviations from the median, multiplied by a constant (1.4826) to yield asymptotically normal consistency.

```
mad(nyfs1$bmi)
```

```
[1] 3.26
```

## 7.13 Assessing Skew

A relatively common idea is to assess **skewness**, several measures of which (including the one below, sometimes called type 3 skewness, or Pearson's moment coefficient of skewness) are available. Many models assume a Normal distribution, where, among other things, the data are symmetric around the mean.

Skewness measures asymmetry in the distribution - left skew (mean < median) is indicated by negative skewness values, while right skew (mean > median) is indicated by positive values. The skew value will be near zero for data that follow a Normal distribution.

### 7.13.1 Non-parametric Skew via `skew1`

A simpler measure of skew, sometimes called the **nonparametric skew** and closely related to Pearson's notion of median skewness, falls between -1 and +1 for any distribution. It is just the difference between the mean and the median, divided by the standard deviation.

- Values greater than +0.2 are sometimes taken to indicate fairly substantial right skew, while values below -0.2 indicate fairly substantial left skew.

```
(mean(nyfs1$bmi) - median(nyfs1$bmi))/sd(nyfs1$bmi)
```

```
[1] 0.269
```

There is a function in the `Love-boost.R` script called `skew1` that can be used to do these calculations, so long as the variable has no missing data.

```
skew1(nyfs1$bmi)
```

```
[1] 0.269
```

The Wikipedia page on skewness, from which some of this material is derived, provides definitions for several other skewness measures.

## 7.14 Assessing Kurtosis (Heavy-Tailedness)

Another measure of a distribution's shape that can be found in the `psych` library is the **kurtosis**. Kurtosis is an indicator of whether the distribution is heavy-tailed or light-tailed as compared to a Normal distribution. Positive kurtosis means more of the variance is due to outliers - unusual points far away from the mean relative to what we might expect from a Normally distributed data set with the same standard deviation.

- A Normal distribution will have a kurtosis value near 0, a distribution with similar tail behavior to what we would expect from a Normal is said to be *mesokurtic*
- Higher kurtosis values (meaningfully higher than 0) indicate that, as compared to a Normal distribution, the observed variance is more the result of extreme outliers (i.e. heavy tails) as opposed to being the result of more modest sized deviations from the mean. These heavy-tailed, or outlier prone, distributions are sometimes called *leptokurtic*.
- Kurtosis values meaningfully lower than 0 indicate light-tailed data, with fewer outliers than we'd expect in a Normal distribution. Such distributions are sometimes referred to as *platykurtic*, and include distributions without outliers, like the Uniform distribution.

Here's a table:

Fewer outliers than a Normal	Approximately Normal	More outliers than a Normal
Light-tailed <i>platykurtic</i> (kurtosis < 0)	"Normalish" <i>mesokurtic</i> (kurtosis = 0)	Heavy-tailed <i>leptokurtic</i> (kurtosis > 0)

```
psych::kurtosi(nyfs1$bmi)
```

```
[1] 1.97
```

### 7.14.1 The Standard Error of the Sample Mean

The **standard error** of the sample mean, which is the standard deviation divided by the square root of the sample size:

```
sd(nyfs1$bmi)/sqrt(length(nyfs1$bmi))
```

```
[1] 0.108
```

## 7.15 The `describe` function in the `Hmisc` library

The `Hmisc` library has lots of useful functions. It's named for its main developer, Frank Harrell. The `describe` function in `Hmisc` knows enough to separate numerical from categorical variables, and give you separate (and detailed) summaries for each.

- For a categorical variable, it provides counts of total observations (n), the number of missing values, and the number of unique categories, along with counts and percentages falling in each category.
- For a numerical variable, it provides:
  - counts of total observations (n), the number of missing values, and the number of unique values
  - an Info value for the data, which indicates how continuous the variable is (a score of 1 is generally indicative of a completely continuous variable with no ties, while scores near 0 indicate lots of ties, and very few unique values)
  - the sample Mean
  - many sample percentiles (quantiles) of the data, specifically (5, 10, 25, 50, 75, 90, 95, 99)

- either a complete table of all observed values, with counts and percentages (if there are a modest number of unique values), or
- a table of the five smallest and five largest values in the data set, which is useful for range checking

```
Hmisc::describe(nyfs1)
```

nyfs1

		7 Variables		1416 Observations			
<hr/>							
subject.id		n	missing	distinct	Info	Mean	Gmd
	1416	0		1416	1	72703	525.3
	.25	.50		.75	.90	.95	
	72313	72698		73096	73331	73414	
<hr/>							
lowest : 71918 71919 71921 71922 71923, highest: 73488 73489 73490 73491 73492							
<hr/>							
sex		n	missing	distinct			
	1416	0		2			
<hr/>							
Value		Female	Male				
Frequency	707		709				
Proportion	0.499		0.501				
<hr/>							
age.exam		n	missing	distinct	Info	Mean	Gmd
	1416	0		14	0.994	8.855	4.235
	.25	.50		.75	.90	.95	
	6	9		12	14	15	
<hr/>							
Value	3	4	5	6	7	8	9
Frequency	97	111	119	129	123	120	90
Proportion	0.069	0.078	0.084	0.091	0.087	0.085	0.064
	10	11	12				
	109	102	108				
<hr/>							
Value	13	14	15	16			
Frequency	113	104	85	6			
Proportion	0.080	0.073	0.060	0.004			
<hr/>							
bmi		n	missing	distinct	Info	Mean	Gmd
	1416	0		191	1	18.8	4.321
	.25	.50		.75	.90	.95	
	15.80	17.70		20.90	24.45	27.00	
<hr/>							
lowest : 11.9 12.6 12.7 12.9 13.0, highest: 34.6 34.7 35.9 37.0 38.8							
<hr/>							
bmi.cat		n	missing	distinct			
	1416	0		4			
<hr/>							
Value	1	Underweight	2	Normal weight	3	Overweight	4
Frequency		42		926		237	211
Proportion		0.030		0.654		0.167	0.149

```
-----
waist.circ
  n  missing distinct      Info      Mean      Gmd      .05      .10
  1416       0      462        1    65.29    14.23    49.30   51.10
  .25       .50      .75        .90      .95
  55.00    63.00    72.93     82.35    90.40

lowest : 42.5 43.4 44.1 44.4 44.7, highest: 108.4 108.5 110.4 111.0 112.4
-----
triceps.skinfold
  n  missing distinct      Info      Mean      Gmd      .05      .10
  1416       0      236        1    13.37    6.279    6.775    7.400
  .25       .50      .75        .90      .95
  9.000   11.800   16.600    21.750   25.600

lowest : 4.0 4.6 4.9 5.0 5.2, highest: 34.3 34.8 36.0 36.2 38.2
-----
```

More on the `Info` value in `Hmisc::describe` is available here

## 7.16 xda from GitHub for numerical summaries for exploratory data analysis

```
## next two commands needed if xda is not already installed
library(devtools)
install_github("ujjwalkarn/xdar")
```

Skipping install of 'xda' from a github remote, the SHA1 (fb68f0da) has not changed since last install.  
Use `force = TRUE` to force installation

```
xda::numSummary(nyfs1)
```

	n	mean	sd	max	min	range	nunique	
subject.id	1416	72702.70	454.75	73492.0	71918.0	1574.0	1416	
age.exam	1416	8.86	3.68	16.0	3.0	13.0	14	
bmi	1416	18.80	4.08	38.8	11.9	26.9	191	
waist.circ	1416	65.29	12.85	112.4	42.5	69.9	462	
triceps.skinfold	1416	13.37	5.83	38.2	4.0	34.2	236	
		nzeros	iqr	lowerbound	upperbound	outlier	kurtosis	
subject.id	0	784.0	71136.75	74272.2	0	-1.193		
age.exam	0	6.0	-3.00	21.0	0	-1.198		
bmi	0	5.1	8.15	28.5	53	1.973		
waist.circ	0	17.9	28.15	99.8	22	0.384		
triceps.skinfold	0	7.6	-2.40	28.0	31	1.149		
		skewness	mode	miss	miss%	1%	5%	25%
subject.id	0.00815	71918.0	0	0	71933.1	71993.75	72312.8	
age.exam	0.08202	6.0	0	0	3.0	3.00	6.0	
bmi	1.34804	15.5	0	0	13.5	14.30	15.8	
waist.circ	0.85106	55.4	0	0	46.1	49.30	55.0	
triceps.skinfold	1.15791	8.0	0	0	5.6	6.77	9.0	
		50%	75%	95%	99%			
subject.id	72697.5	73096.2	73414.2	73478				

age.exam	9.0	12.0	15.0	15
bmi	17.7	20.9	27.0	32
waist.circ	63.0	72.9	90.4	102
triceps.skinfold	11.8	16.6	25.6	31

Most of the elements of this `numSummary` should be familiar. Some new pieces include:

- `nunique` = number of unique values
- `nzeroes` = number of zeroes
- `noutlier` = number of outliers (using a standard that isn't entirely transparent to me)
- `miss` = number of rows with missing value
- `miss%` = percentage of total rows with missing values ( $(\text{miss}/n)*100$ )
- `5%` = 5th percentile value of that variable (value below which 5 percent of the observations may be found)

```
xda::charSummary(nyfs1)
```

	n	miss	miss%	unique
sex	1416	0	0	2
bmi.cat	1416	0	0	4

top5levels:count

sex	Male:709, Female:707
bmi.cat	2 Normal weight:926, 3 Overweight:237, 4 Obese:211, 1 Underweight:42

The `top5levels:count` provides the top 5 unique values for each variable, sorted by their counts.

## 7.17 What Summaries to Report

It is usually helpful to focus on the shape, center and spread of a distribution. Bock, Velleman and DeVeaux provide some useful advice:

- If the data are skewed, report the median and IQR (or the three middle quantiles). You may want to include the mean and standard deviation, but you should point out why the mean and median differ. The fact that the mean and median do not agree is a sign that the distribution may be skewed. A histogram will help you make that point.
- If the data are symmetric, report the mean and standard deviation, and possibly the median and IQR as well.
- If there are clear outliers and you are reporting the mean and standard deviation, report them with the outliers present and with the outliers removed. The differences may be revealing. The median and IQR are not likely to be seriously affected by outliers.

# Chapter 8

## Assessing Normality

Data are well approximated by a Normal distribution if the shape of the data's distribution is a good match for a Normal distribution with mean and standard deviation equal to the sample statistics.

- the data are symmetrically distributed about a single peak, located at the sample mean
- the spread of the distribution is well characterized by a Normal distribution with standard deviation equal to the sample standard deviation
- the data show outlying values (both in number of candidate outliers, and size of the distance between the outliers and the center of the distribution) that are similar to what would be predicted by a Normal model.

We have several tools for assessing Normality of a single batch of data, including:

- a histogram with superimposed Normal distribution
- histogram variants (like the boxplot) which provide information on the center, spread and shape of a distribution
- the Empirical Rule for interpretation of a standard deviation
- a specialized *normal Q-Q plot* (also called a normal probability plot or normal quantile-quantile plot) designed to reveal differences between a sample distribution and what we might expect from a normal distribution of a similar number of values with the same mean and standard deviation

### 8.1 Empirical Rule Interpretation of the Standard Deviation

For a set of measurements that follows a Normal distribution, the interval:

- Mean  $\pm$  Standard Deviation contains approximately 68% of the measurements;
- Mean  $\pm$  2(Standard Deviation) contains approximately 95% of the measurements;
- Mean  $\pm$  3(Standard Deviation) contains approximately all (99.7%) of the measurements.

Again, most data sets do not follow a Normal distribution. We will occasionally think about transforming or re-expressing our data to obtain results which are better approximated by a Normal distribution, in part so that a standard deviation can be more meaningful.

For the BMI data we have been studying, here again are some summary statistics...

```
mosaic::favstats(nyfs1$bmi)
```

min	Q1	median	Q3	max	mean	sd	n	missing
11.9	15.8	17.7	20.9	38.8	18.8	4.08	1416	0

The mean is 18.8 and the standard deviation is 4.08, so if the data really were Normally distributed, we'd expect to see:

- About 68% of the data in the range (14.72, 22.88). In fact, 1074 of the 1416 BMI values are in this range, or 75.8%.
- About 95% of the data in the range (10.64, 26.96). In fact, 1344 of the 1416 BMI values are in this range, or 94.9%.
- About 99.7% of the data in the range (6.56, 31.04). In fact, 1393 of the 1416 BMI values are in this range, or 98.4%.

So, based on this Empirical Rule approximation, do the BMI data seem to be well approximated by a Normal distribution?

## 8.2 Describing Outlying Values with Z Scores

The maximum body-mass index value here is 38.8. One way to gauge how extreme this is (or how much of an outlier it is) uses that observation's **Z score**, the number of standard deviations away from the mean that the observation falls.

Here, the maximum value, 38.8 is 4.9 standard deviations above the mean, and thus has a Z score of 4.9.

A negative Z score would indicate a point below the mean, while a positive Z score indicates, as we've seen, a point above the mean. The minimum body-mass index, 11.9 is 1.69 standard deviations *below* the mean, so it has a Z score of -1.7.

Recall that the Empirical Rule suggests that if a variable follows a Normal distribution, it would have approximately 95% of its observations falling inside a Z score of (-2, 2), and 99.74% falling inside a Z score range of (-3, 3).

### 8.2.1 Fences and Z Scores

Note the relationship between the fences (Tukey's approach to identifying points which fall within the whiskers of a boxplot, as compared to candidate outliers) and the Z scores.

The upper inner fence in this case falls at 28.55, which indicates a Z score of 2.4, while the lower inner fence falls at 8.15, which indicates a Z score of -2.6. It is neither unusual nor inevitable for the inner fences to fall at Z scores near -2.0 and +2.0.

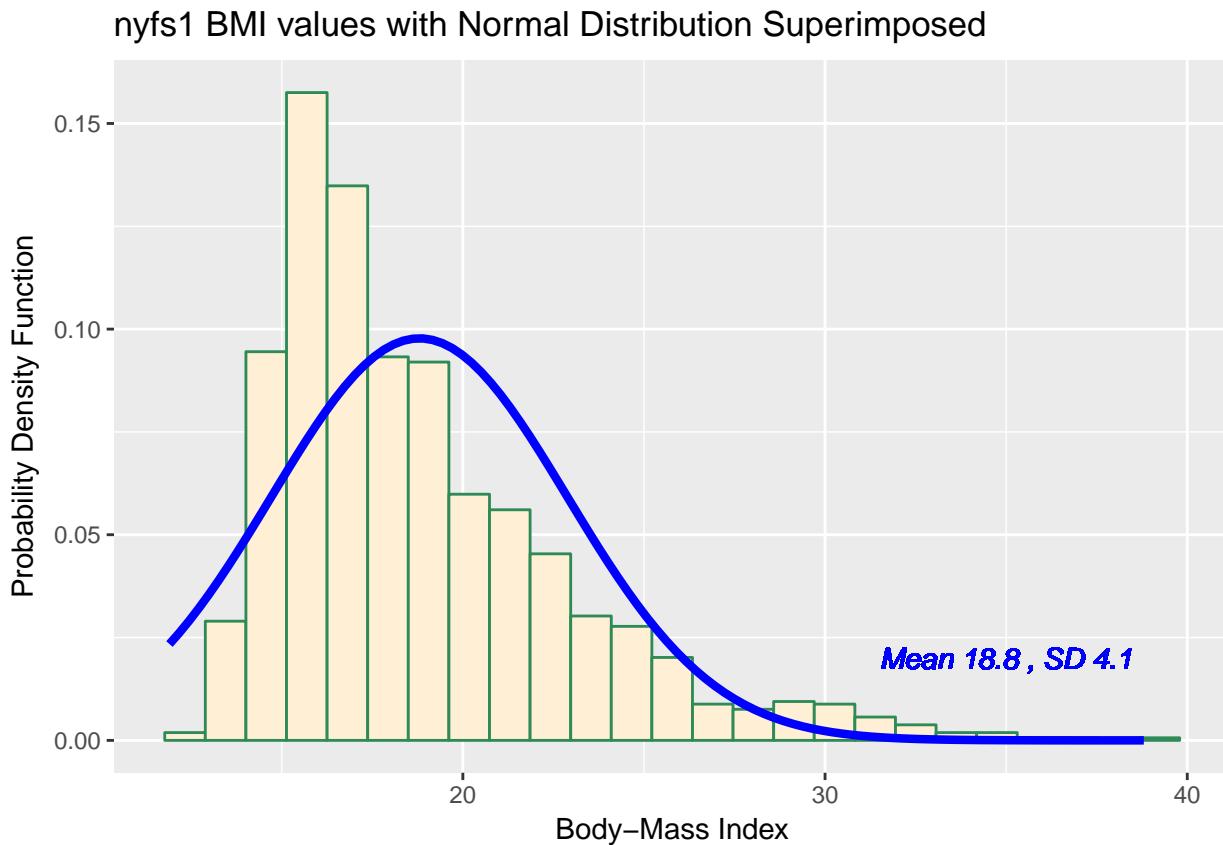
## 8.3 Comparing a Histogram to a Normal Distribution

Most of the time, when we want to understand whether our data are well approximated by a Normal distribution, we will use a graph to aid in the decision.

One option is to build a histogram with a Normal density function (with the same mean and standard deviation as our data) superimposed. This is one way to help visualize deviations between our data and what might be expected from a Normal distribution.

```
ggplot(nyfs1, aes(x=bmi)) +
  geom_histogram(aes(y = ..density..), bins=25, fill = "papayawhip", color = "seagreen") +
  stat_function(fun = dnorm,
               args = list(mean = mean(nyfs1$bmi), sd = sd(nyfs1$bmi)),
               lwd = 1.5, col = "blue") +
  geom_text(aes(label = paste("Mean", round(mean(nyfs1$bmi),1),
                  ", SD", round(sd(nyfs1$bmi),1))),
```

```
x = 35, y = 0.02, color="blue", fontface = "italic") +
  labs(title = "nyfs1 BMI values with Normal Distribution Superimposed",
       x = "Body-Mass Index", y = "Probability Density Function")
```



Does it seem as though the Normal model (as shown in the blue density curve) is an effective approximation to the observed distribution shown in the bars of the histogram?

We'll return shortly to the questions:

- Does a Normal distribution model fit our data well? *and*
- If the data aren't Normal, but we want to use a Normal model anyway, what should we do?

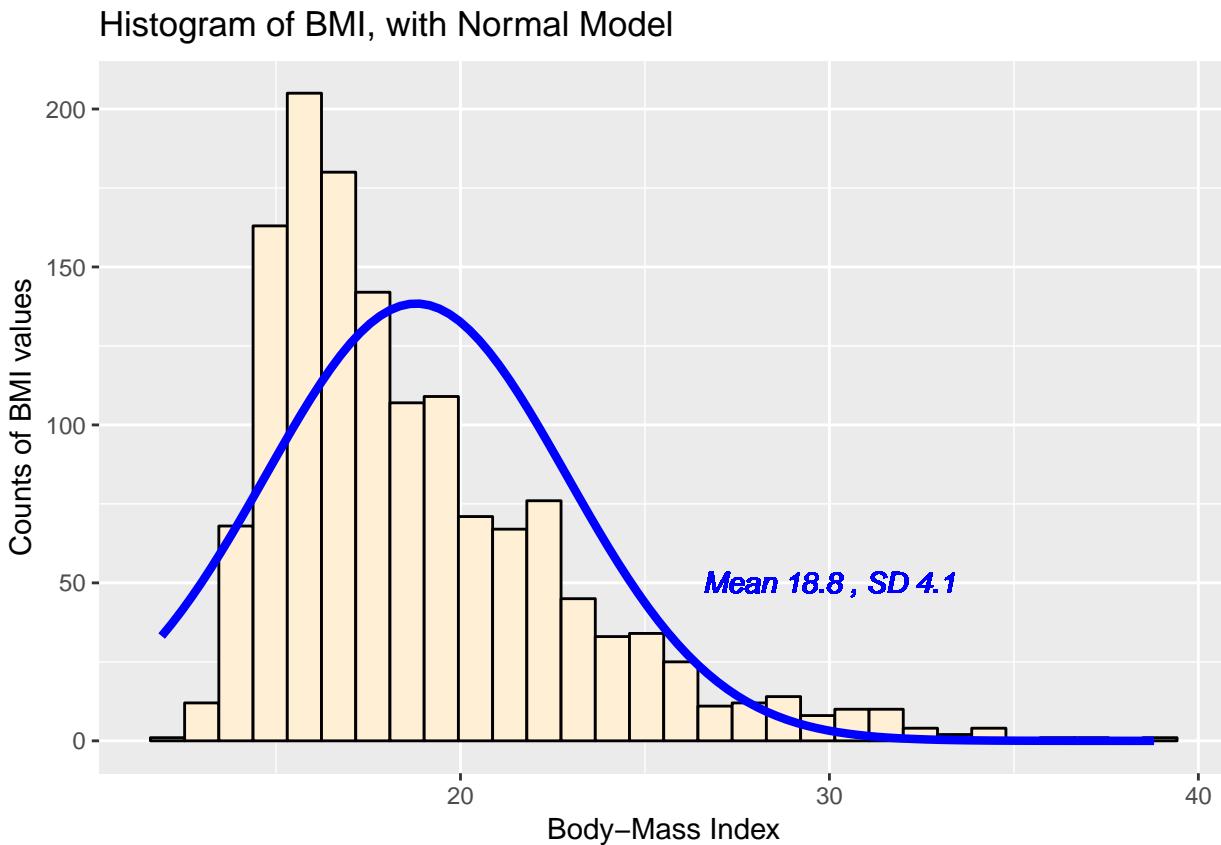
### 8.3.1 Histogram of BMI with Normal model (with Counts)

But first, we'll demonstrate an approach to building a histogram of counts (rather than a probability density) and then superimposing a Normal model.

```
## ggplot of counts of bmi with Normal model superimposed
## Source: https://stat.ethz.ch/pipermail/r-help/2009-September/403220.html

ggplot(nyfs1, aes(x = bmi)) +
  geom_histogram(bins = 30, fill = "papayawhip", color = "black") +
  stat_function(fun = function(x, mean, sd, n)
    n * dnorm(x = x, mean = mean, sd = sd),
    args = with(nyfs1,
               c(mean = mean(bmi), sd = sd(bmi), n = length(bmi))),
    col = "blue", lwd = 1.5) +
```

```
geom_text(aes(label = paste("Mean", round(mean(nyfs1$bmi),1),
                  ", SD", round(sd(nyfs1$bmi),1))),
          x = 30, y = 50, color="blue", fontface = "italic") +
  labs(title = "Histogram of BMI, with Normal Model",
       x = "Body-Mass Index", y = "Counts of BMI values")
```

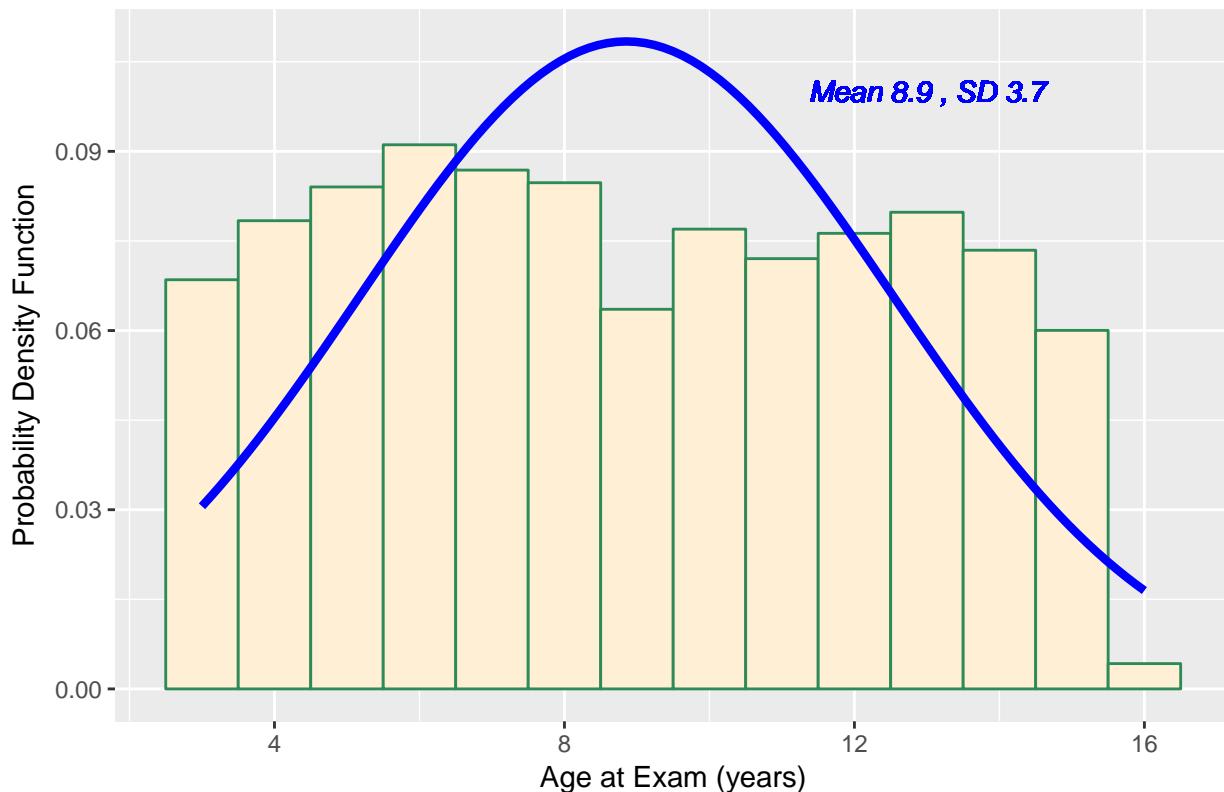


## 8.4 Does a Normal model work well for the Ages?

Now, suppose we instead look at the `age.exam` data. Do these data appear to follow a Normal distribution?

```
ggplot(nyfs1, aes(x=age.exam)) +
  geom_histogram(aes(y = ..density..), binwidth=1,
                 fill = "papayawhip", color = "seagreen") +
  stat_function(fun = dnorm,
                args = list(mean = mean(nyfs1$age.exam),
                            sd = sd(nyfs1$age.exam)),
                lwd = 1.5, col = "blue") +
  geom_text(aes(label = paste("Mean", round(mean(nyfs1$age.exam),1),
                  ", SD", round(sd(nyfs1$age.exam),1))),
          x = 13, y = 0.1, color="blue", fontface = "italic") +
  labs(title = "nyfs1 Age values with Normal Distribution Superimposed",
       x = "Age at Exam (years)", y = "Probability Density Function")
```

### nyfs1 Age values with Normal Distribution Superimposed



```
mosaic::favstats(nyfs1$age.exam)
```

```
min Q1 median Q3 max mean sd n missing
3 6 9 12 16 8.86 3.68 1416 0
```

The mean is 8.86 and the standard deviation is 3.68 so if the `age.exam` data really were Normally distributed, we'd expect to see:

- About 68% of the data in the range (5.17, 12.54). In fact, 781 of the 1416 Age values are in this range, or 55.2%.
- About 95% of the data in the range (1.49, 16.22). In fact, 1416 of the 1416 Age values are in this range, or 100%.
- About 99.7% of the data in the range (-2.19, 19.9). In fact, 1416 of the 1416 Age values are in this range, or 100%.

How does the Normal approximation work for age, according to the Empirical Rule?

There is a function in the `Love-boost.R` script called `Emp_Rule` that can be used to do these calculations, so long as the variable has no missing data.

```
Emp_Rule(nyfs1$bmi)
```

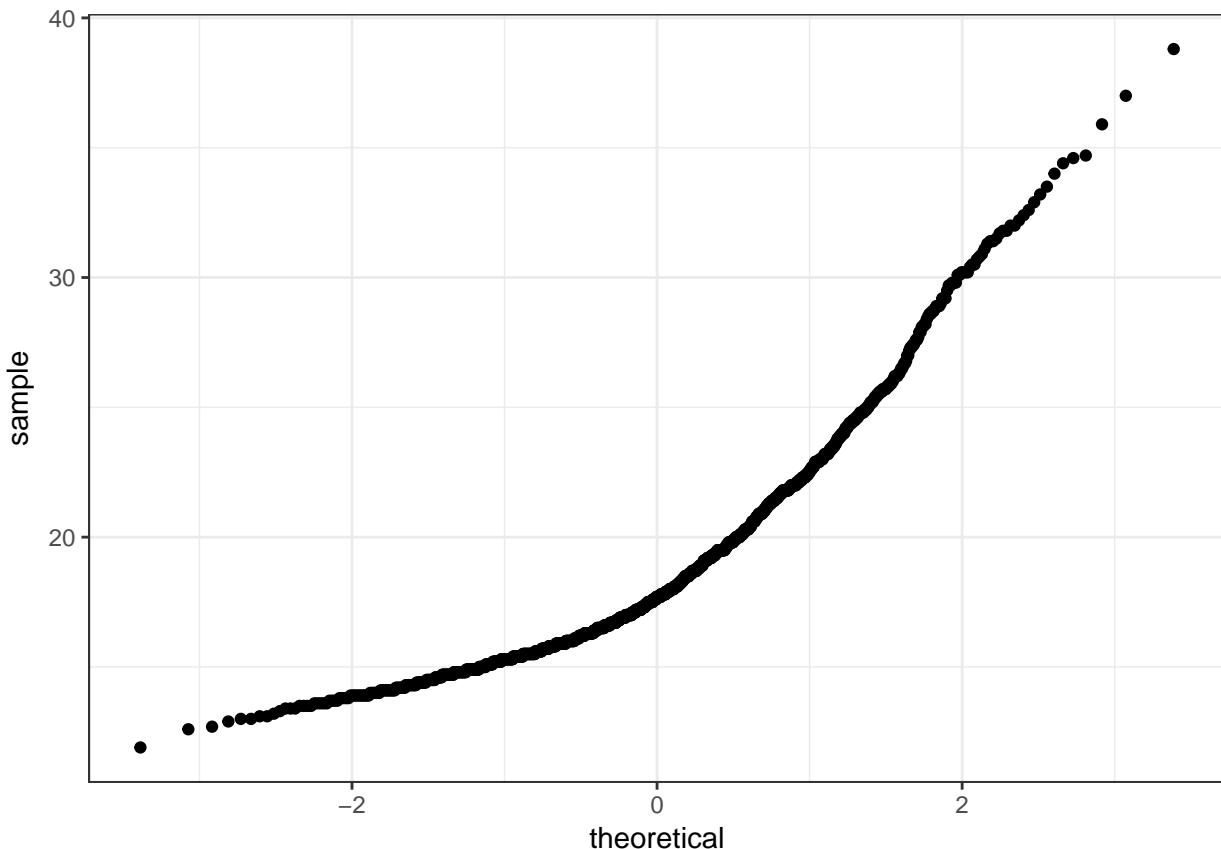
	count	proportion
Mean +/- 1 SD	1074	0.7585
Mean +/- 2 SD	1344	0.9492
Mean +/- 3 SD	1393	0.9838
Entire Data Set	1416	1

## 8.5 The Normal Q-Q Plot

A normal probability plot (or normal quantile-quantile plot) of the BMI results from the `nyfs1` data, developed using `ggplot2` is shown below. In this case, this is a picture of 1416 BMI results. The idea of a normal Q-Q plot is that it plots the observed sample values (on the vertical axis) and then, on the horizontal, the expected or theoretical quantiles that would be observed in a standard normal distribution (a Normal distribution with mean 0 and standard deviation 1) with the same number of observations.

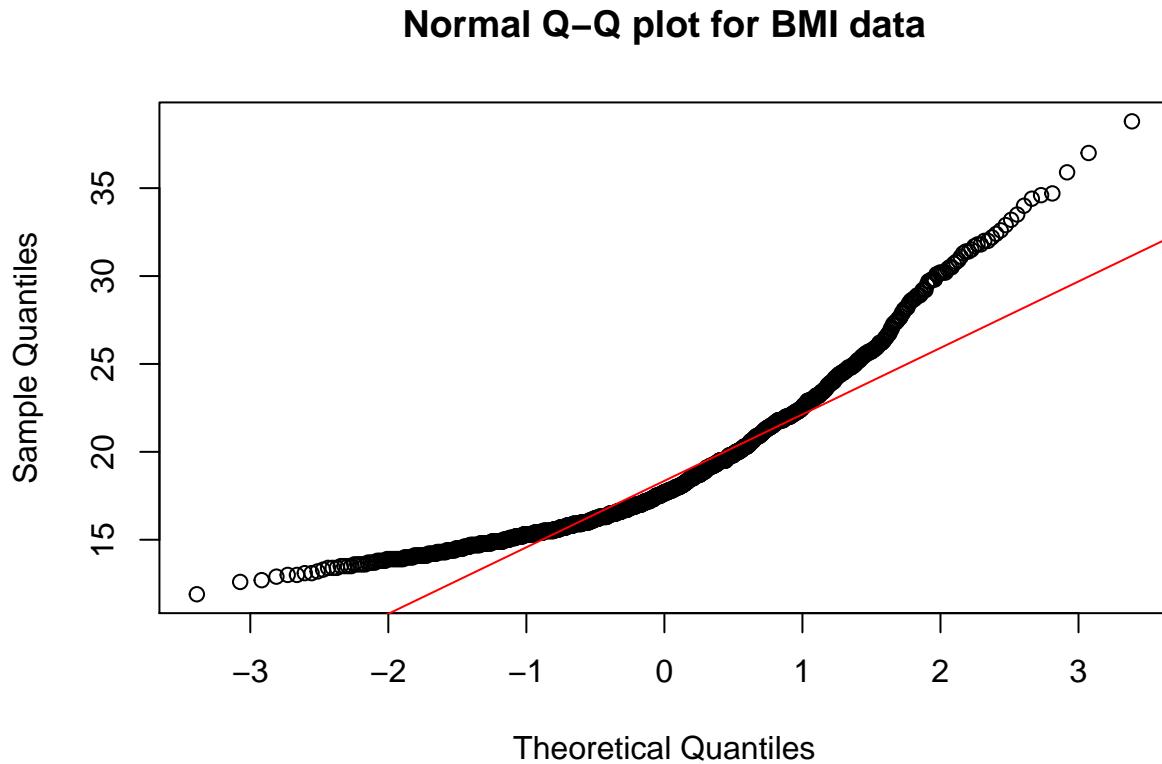
A Normal Q-Q plot will follow a straight line when the data are (approximately) Normally distributed. When the data have a different shape, the plot will reflect that.

```
ggplot(nyfs1, aes(sample = bmi)) +
  geom_point(stat="qq") +
  theme_bw() # eliminate the gray background
```



This is a case where the base graphics approach may be preferable.

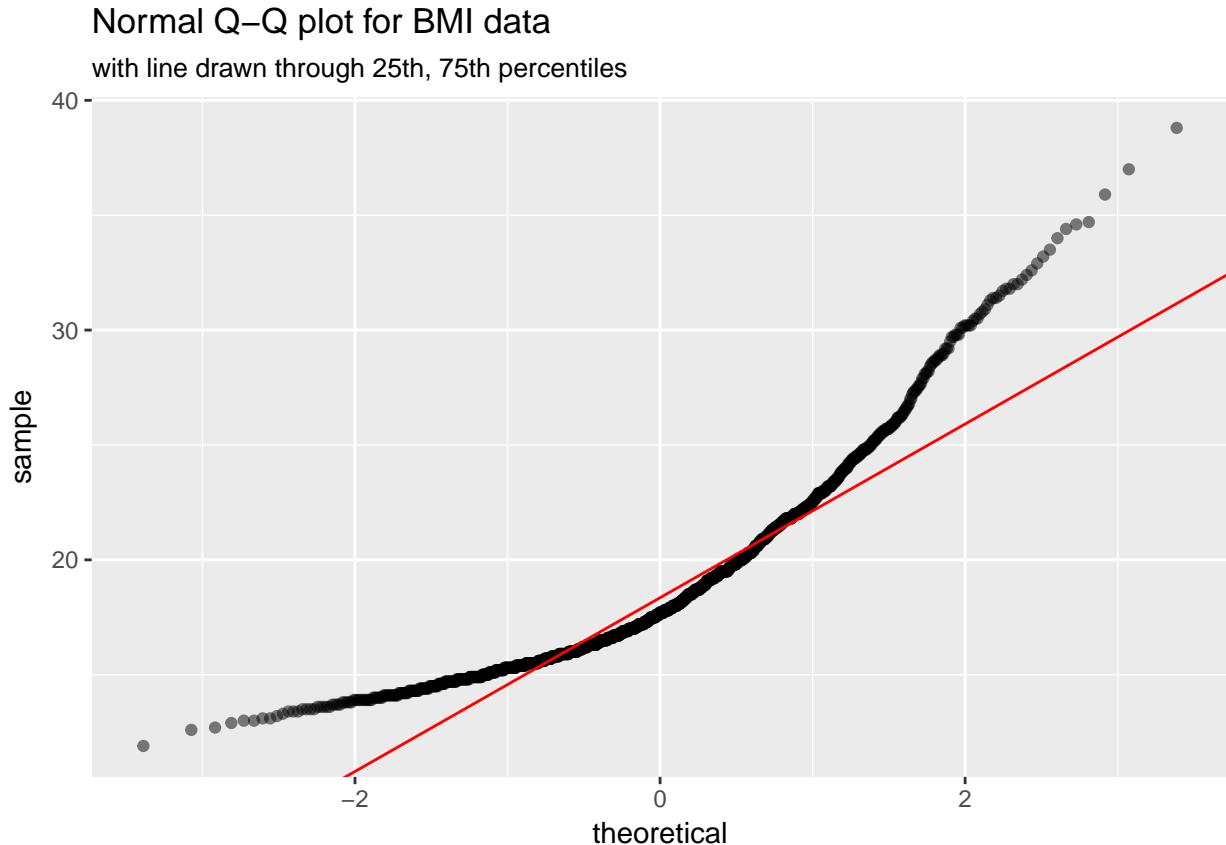
```
qqnorm(nyfs1$bmi, main = "Normal Q-Q plot for BMI data")
qqline(nyfs1$bmi, col = "red")
```



It is possible to get the base graphics result with ggplot2, though. For example, we might use this modification of a response at <https://stackoverflow.com/questions/4357031/qqnorm-and-qqline-in-ggplot2/>

```
dat <- nyfs1 %>% filter(complete.cases(bmi))
y <- quantile(dat$bmi, c(0.25, 0.75))
x <- qnorm(c(0.25, 0.75))
slope <- diff(y)/diff(x)
int <- y[1L] - slope * x[1L]

ggplot(nyfs1, aes(sample = bmi)) +
  geom_qq(alpha = 0.5) +
  geom_abline(slope = slope, intercept = int, col = "red") +
  labs(title = "Normal Q-Q plot for BMI data",
       subtitle = "with line drawn through 25th, 75th percentiles")
```



```
rm(x, y, slope, int, dat)
```

## 8.6 Interpreting the Normal Q–Q Plot

The purpose of a Normal Q–Q plot is to help point out distinctions from a Normal distribution. A Normal distribution is symmetric and has certain expectations regarding its tails. The Normal Q–Q plot can help us identify data as - well approximated by a Normal distribution, or not because of - skew (including distinguishing between right skew and left skew) - behavior in the tails (which could be heavy-tailed [more outliers than expected] or light-tailed)

### 8.6.1 Data from a Normal distribution shows up as a straight line in a Normal Q–Q plot

We'll demonstrate the looks that we can obtain from a Normal Q–Q plot in some simulations. First, here is an example of a Normal Q–Q plot, and its associated histogram, for a sample of 200 observations simulated from a Normal distribution.

```
set.seed(123431) # so the results can be replicated

# simulate 200 observations from a Normal(20, 5) distribution and place them
# in the d variable within the temp.1 data frame
temp.1 <- data.frame(d = rnorm(200, mean = 20, sd = 5))

# left plot - basic Normal Q–Q plot of simulated data
```

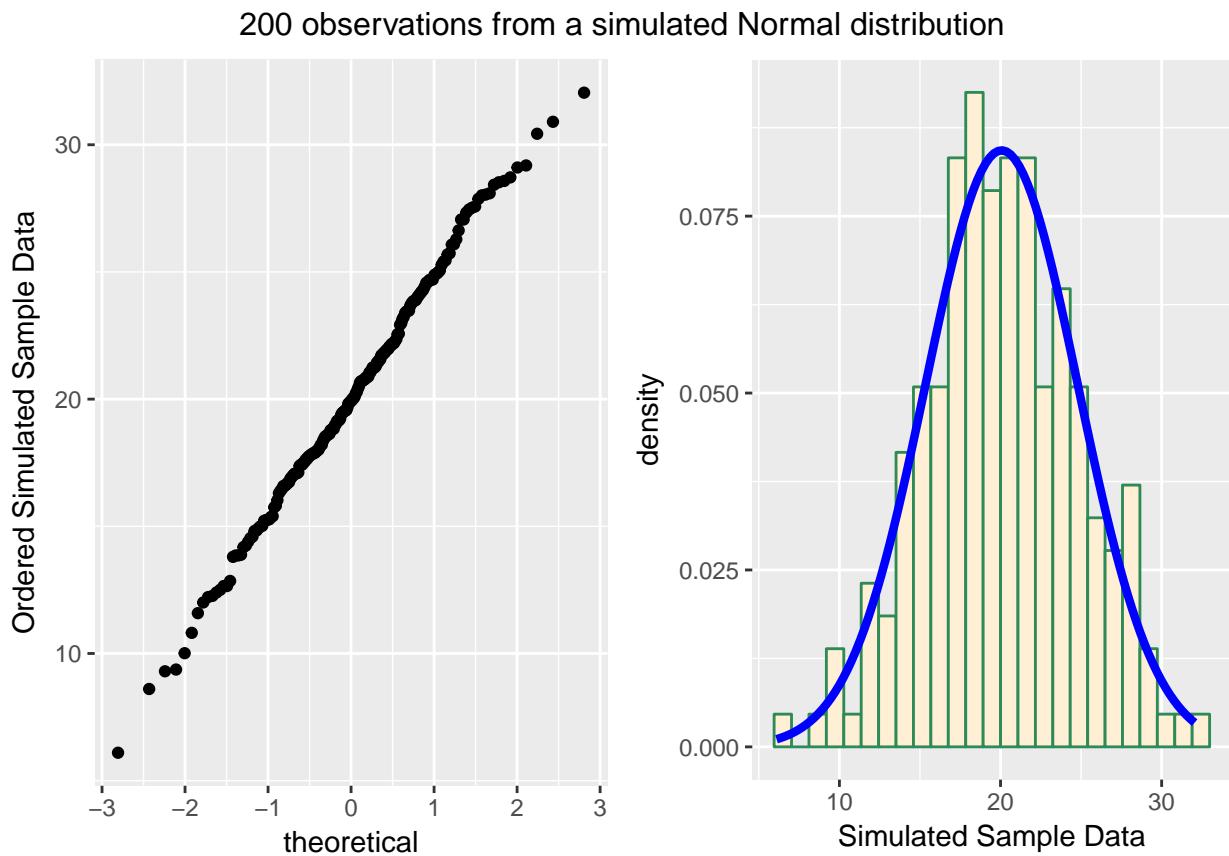
```

p1 <- ggplot(temp.1, aes(sample = d)) +
  geom_point(stat="qq") +
  labs(y = "Ordered Simulated Sample Data")

# right plot - histogram with superimposed normal distribution
p2 <- ggplot(temp.1, aes(x = d)) +
  geom_histogram(aes(y = ..density..),
                 bins=25, fill = "papayawhip", color = "seagreen") +
  stat_function(fun = dnorm,
                args = list(mean = mean(temp.1$d),
                            sd = sd(temp.1$d)),
                lwd = 1.5, col = "blue") +
  labs(x = "Simulated Sample Data")

gridExtra::grid.arrange(p1, p2, ncol=2,
                       top ="200 observations from a simulated Normal distribution")

```

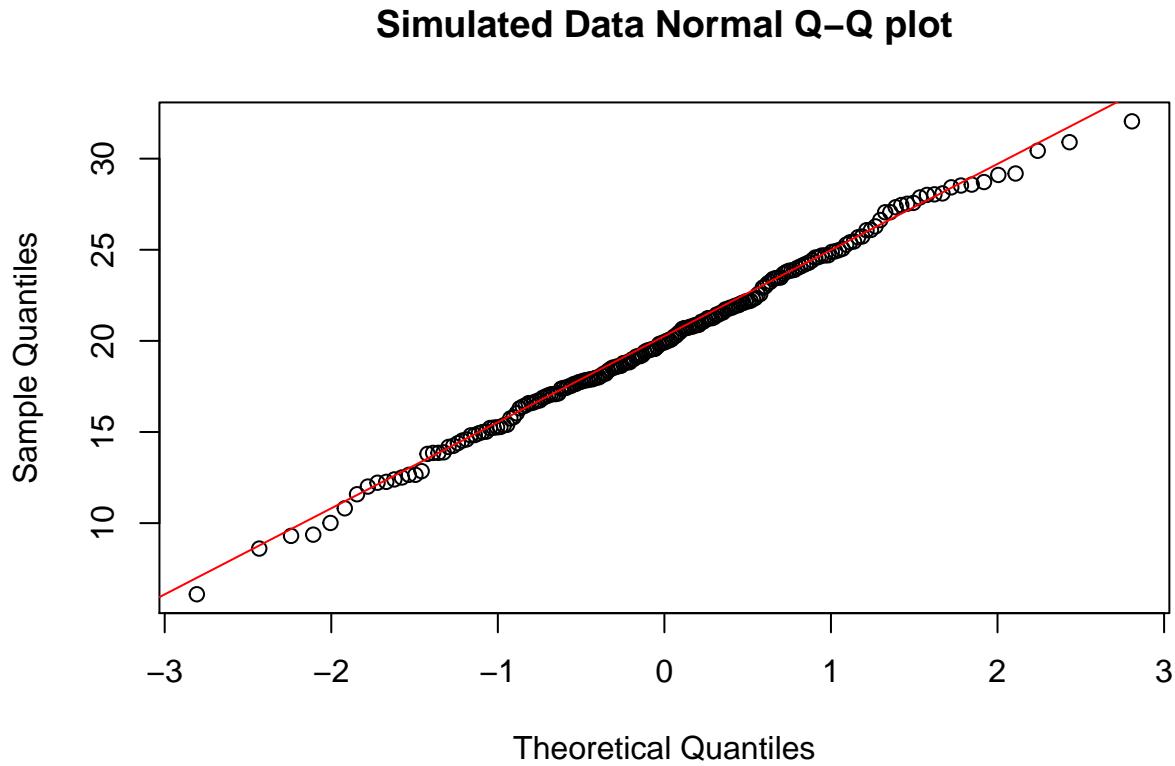


And here is another look at that same simulated data...

```

qqnorm(temp.1$d, main = "Simulated Data Normal Q-Q plot")
qqline(temp.1$d, col = "red")

```



So, what are the characteristics of this simulation? The data appear to be well-modeled by the Normal distribution, because:

- the points on the Normal Q-Q plot follow a straight line, in particular
- there is no substantial curve (such as we'd see with data that were skewed)
- there is no particularly surprising behavior (curves away from the line) at either tail, so there's no obvious problem with outliers

### 8.6.2 Skew is indicated by monotonic curves in the Normal Q-Q plot

Data that come from a skewed distribution appear to curve away from a straight line in the Q-Q plot.

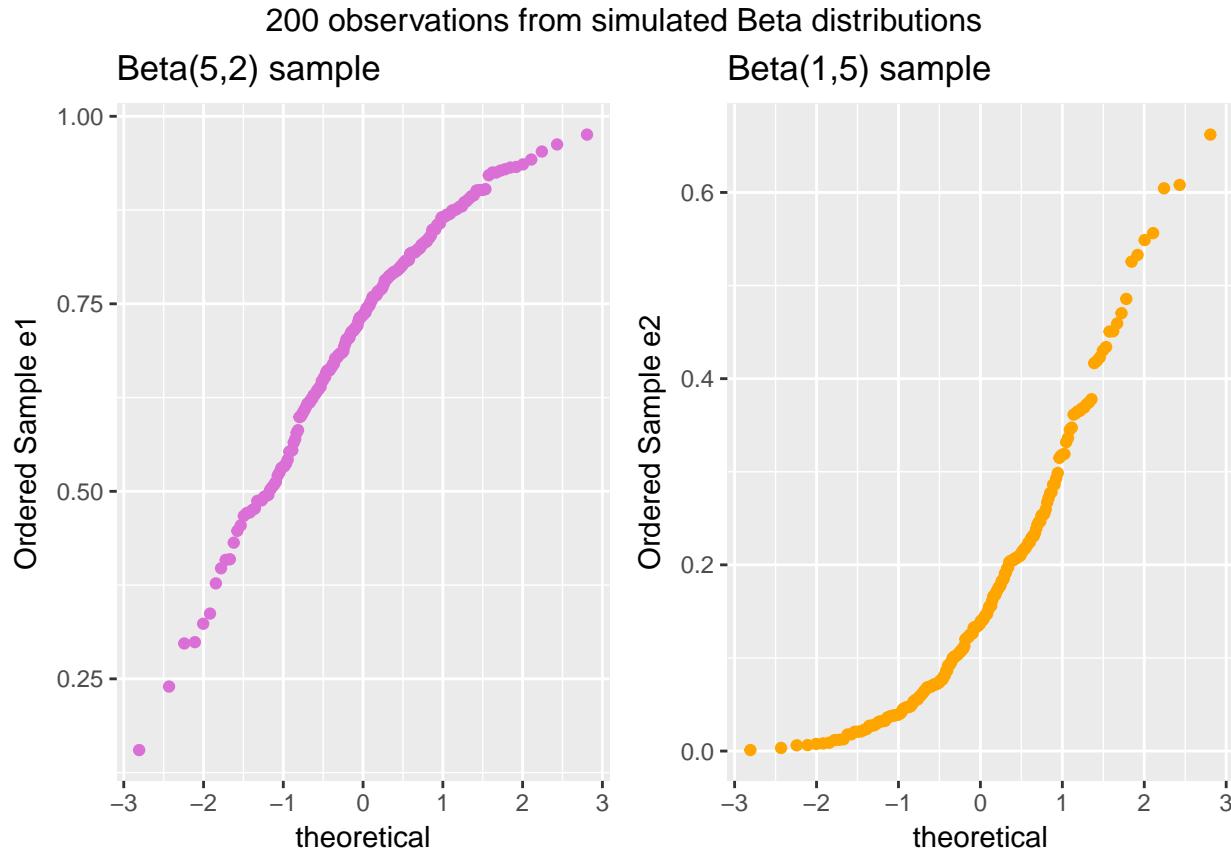
```
set.seed(123431) # so the results can be replicated

# simulate 200 observations from a beta(5, 2) distribution into the e1 variable
# simulate 200 observations from a beta(1, 5) distribution into the e2 variable
temp.2 <- data.frame(e1 = rbeta(200, 5, 2), e2 = rbeta(200, 1, 5))

p1 <- ggplot(temp.2, aes(sample = e1)) +
  geom_point(stat="qq", color = "orchid") +
  labs(y = "Ordered Sample e1", title = "Beta(5,2) sample")

p2 <- ggplot(temp.2, aes(sample = e2)) +
  geom_point(stat="qq", color = "orange") +
  labs(y = "Ordered Sample e2", title = "Beta(1,5) sample")

gridExtra::grid.arrange(p1, p2, ncol=2, top ="200 observations from simulated Beta distributions")
```

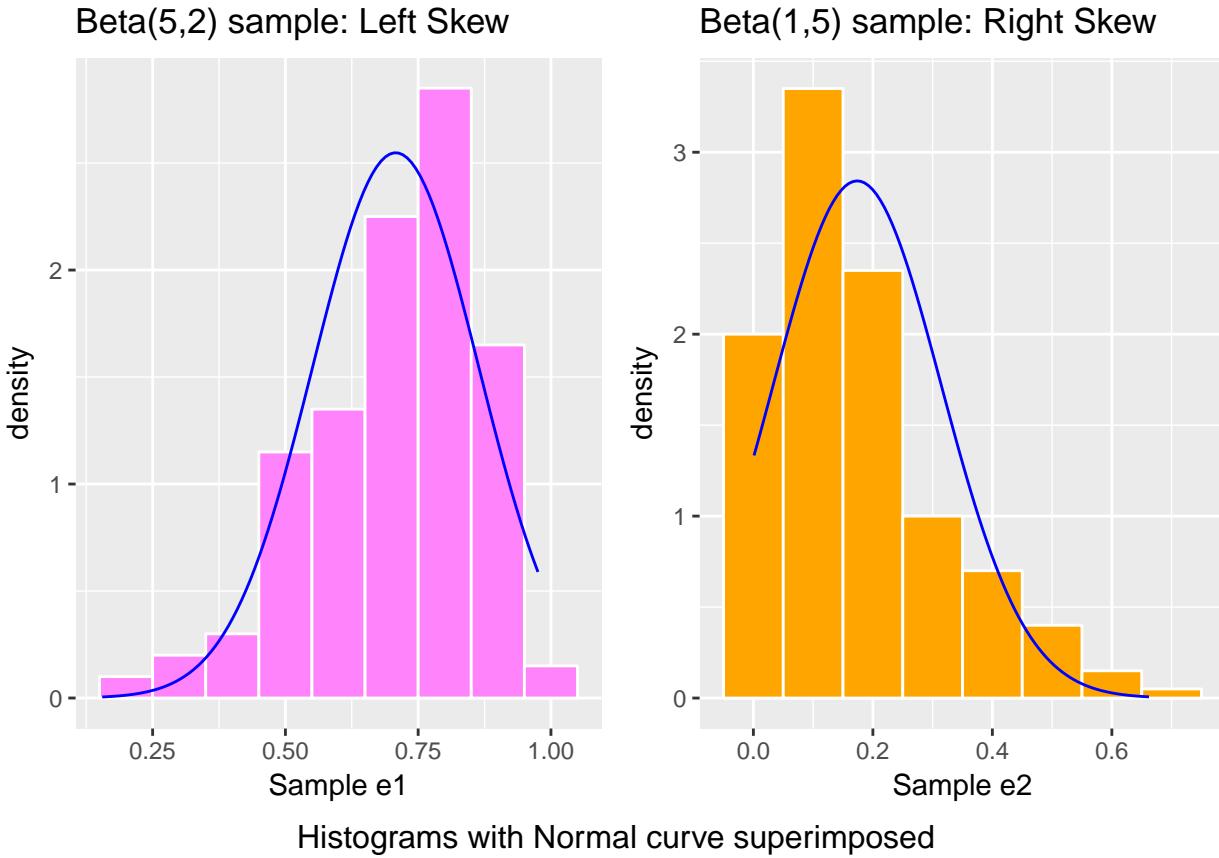


Note the bends away from a straight line in each sample. The non-Normality may be easier to see in a histogram.

```
p1 <- ggplot(temp.2, aes(x = e1)) +
  geom_histogram(aes(y = ..density..),
                 binwidth=0.1, fill = "orchid1", color = "white") +
  stat_function(fun = dnorm,
                args = list(mean = mean(temp.2$e1),
                            sd = sd(temp.2$e1)),
                col = "blue") +
  labs(x = "Sample e1", title = "Beta(5,2) sample: Left Skew")

p2 <- ggplot(temp.2, aes(x = e2)) +
  geom_histogram(aes(y = ..density..),
                 binwidth=0.1, fill = "orange1", color = "white") +
  stat_function(fun = dnorm,
                args = list(mean = mean(temp.2$e2),
                            sd = sd(temp.2$e2)),
                col = "blue") +
  labs(x = "Sample e2", title = "Beta(1,5) sample: Right Skew")

gridExtra::grid.arrange(p1, p2, ncol=2,
bottom ="Histograms with Normal curve superimposed")
```



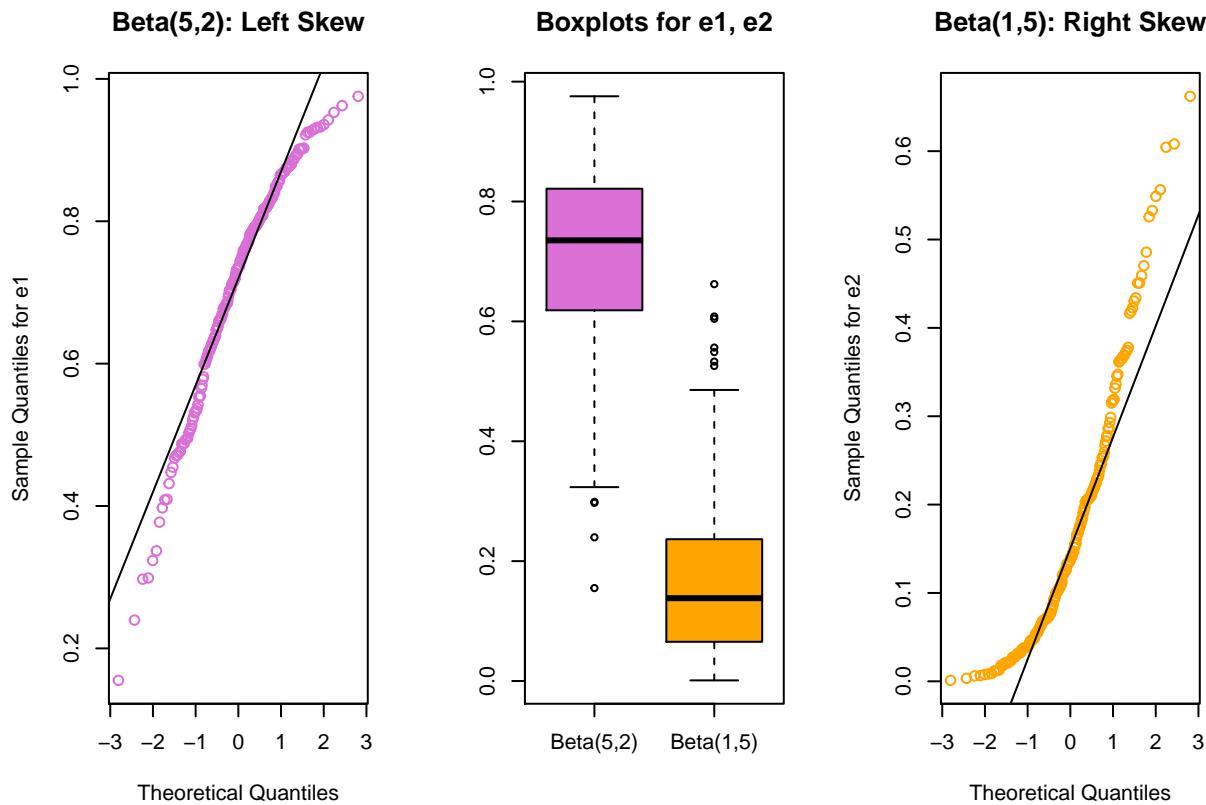
### 8.6.3 Direction of Skew

In each of these pairs of plots, we see the same basic result.

- The left plot (for data e1) shows left skew, with a longer tail on the left hand side and more clustered data at the right end of the distribution.
- The right plot (for data e2) shows right skew, with a longer tail on the right hand side, the mean larger than the median, and more clustered data at the left end of the distribution.

You may want to see the lines to help you see what's happening in the Q-Q plots. You can do this with our fancy approach, or with the qqnorm-qqline combination from base R.

```
par(mfrow=c(1,3))
qqnorm(temp.2$e1, col="orchid", main="Beta(5,2): Left Skew",
      ylab="Sample Quantiles for e1")
qqline(temp.2$e1)
boxplot(temp.2$e1, temp.2$e2, names=c("Beta(5,2)", "Beta(1,5)"),
       col=c("orchid", "orange"), main="Boxplots for e1, e2")
qqnorm(temp.2$e2, col="orange", main="Beta(1,5): Right Skew",
      ylab="Sample Quantiles for e2")
qqline(temp.2$e2)
```



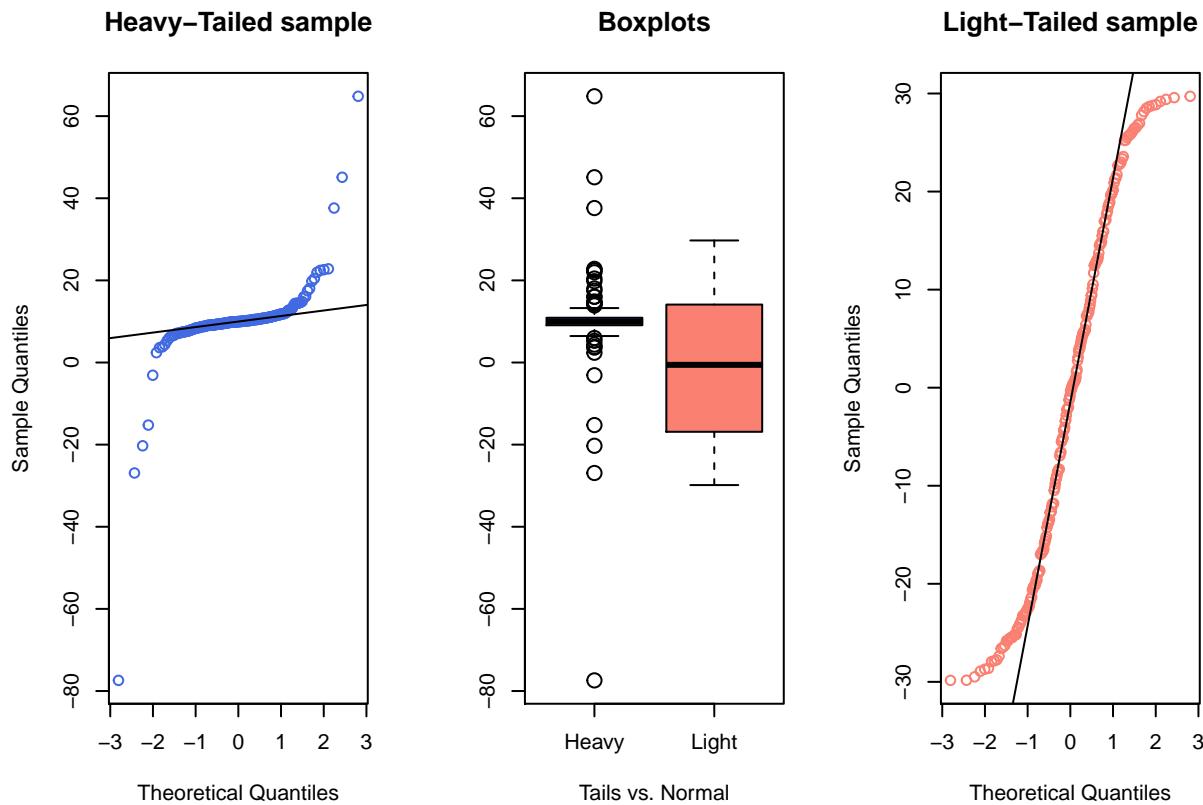
```
par(mfrow=c(1,1))
```

#### 8.6.4 Outlier-proneness is indicated by “s-shaped” curves in a Normal Q-Q plot

- Heavy-tailed but symmetric distributions are indicated by reverse “S”-shapes, as shown on the left below.
- Light-tailed but symmetric distributions are indicated by “S” shapes in the plot, as shown on the right below.

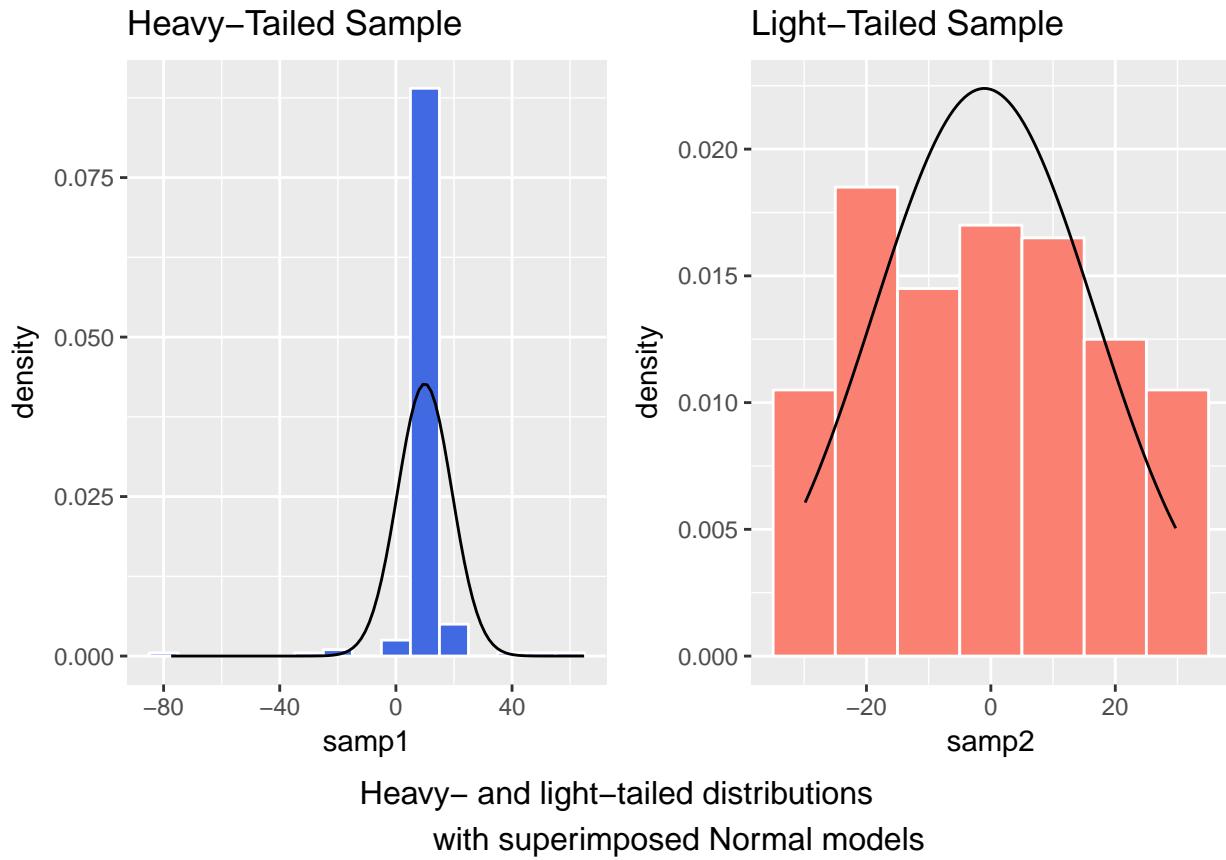
```
set.seed(4311)
# sample 200 observations from each of two probability distributions
samp1 <- rcauchy(200, location=10, scale = 1) # use a Cauchy distribution
samp2 <- runif(200, -30, 30) # a uniform distribution on (-30, 30)

par(mfrow=c(1,3)) ## set up plot window for one row, three columns
qnorm(samp1, col="royalblue", main="Heavy-Tailed sample")
qqline(samp1)
boxplot(samp1, samp2, names=c("Heavy", "Light"), cex=1.5,
        col=c("royalblue", "salmon"), main="Boxplots",
        xlab="Tails vs. Normal")
qnorm(samp2, col="salmon", main="Light-Tailed sample")
qqline(samp2)
```



```
par(mfrow=c(1,1)) ## return to usual plot window
```

And, we can verify these initial conclusions with histograms.

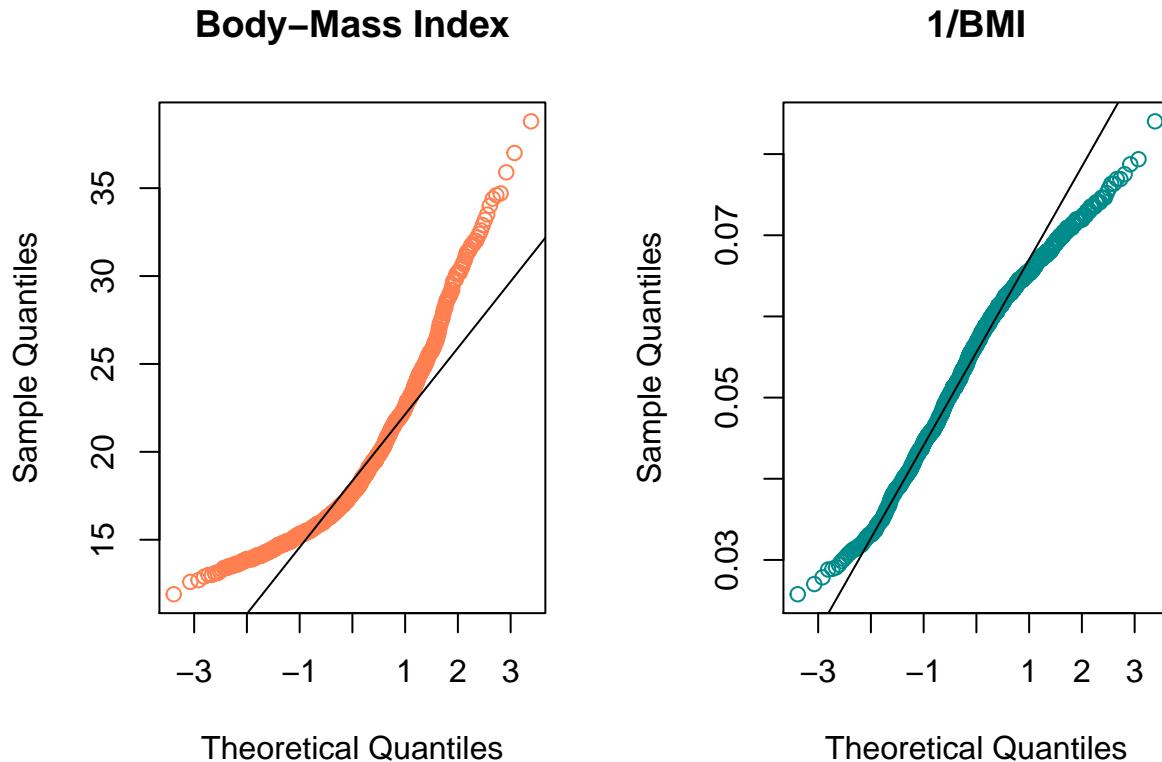


```
rm(samp1, samp2, temp.1, temp.2, temp.3, p1, p2) # cleaning up
```

## 8.7 Does a Normal Distribution Fit the nyfs1 Data Well?

- Skewness is indicated by curves in the Normal Q-Q plot. Compare these two plots - the left is the original BMI data from the NYFS data frame, and the right plot shows the inverse of those values.

```
par(mfrow=c(1,2)) ## set up plot window for one row, two columns
qqnorm(nyfs1$bmi, main="Body-Mass Index", col="coral")
qqline(nyfs1$bmi)
qqnorm(1/(nyfs1$bmi), main="1/BMI", col="darkcyan")
qqline(1/nyfs1$bmi)
```



```
par(mfrow=c(1,1)) ## return to usual plot window
```

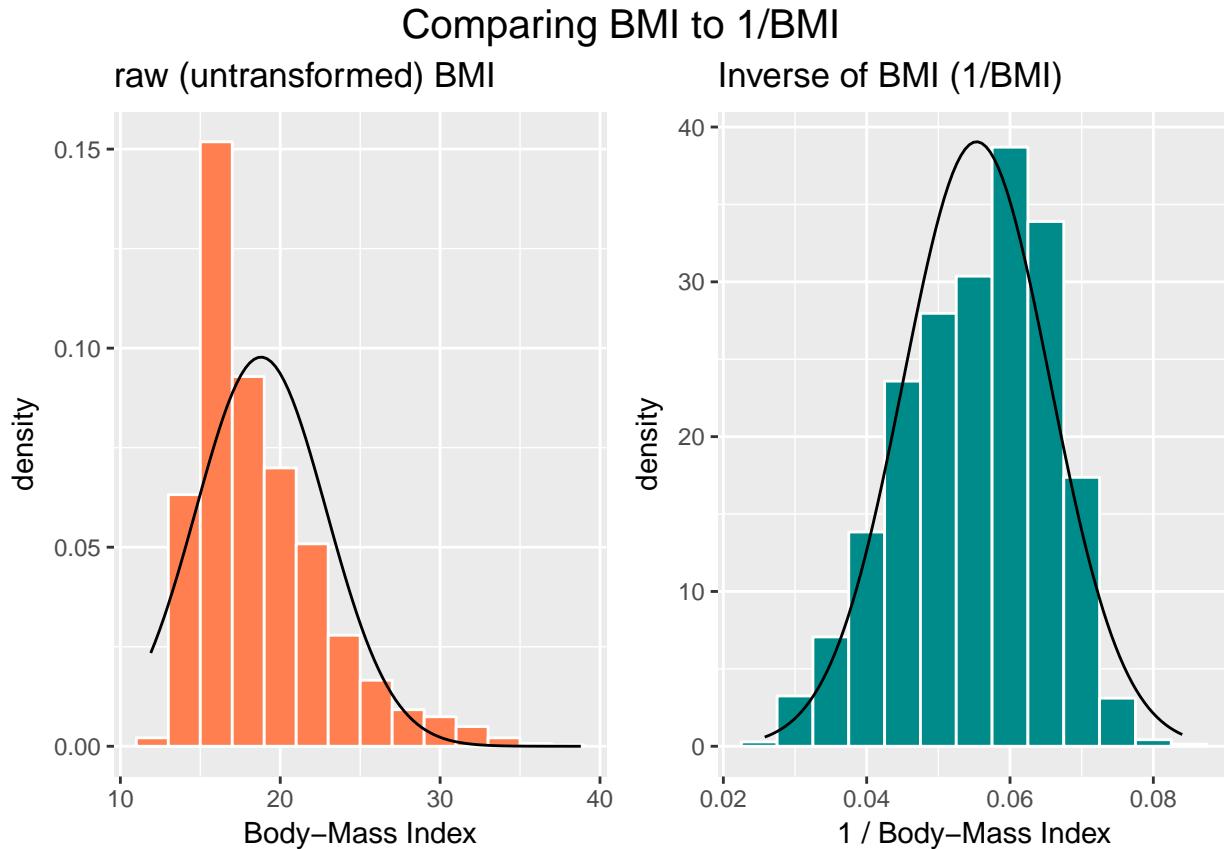
- The left plot shows fairly substantial **right** or *positive* skew
- The right plot shows there's much less skew after the inverse has been taken.
- Our conclusion is that a Normal model is a far better fit to 1/BMI than it is to BMI.

The effect of taking the inverse here may be clearer from the histograms below, with Normal density functions superimposed.

```
p1 <- ggplot(nyfs1, aes(x = bmi)) +
  geom_histogram(aes(y = ..density..),
                 binwidth=2, fill = "coral", color = "white") +
  stat_function(fun = dnorm,
                args = list(mean = mean(nyfs1$bmi), sd = sd(nyfs1$bmi))) +
  labs(x = "Body-Mass Index", title = "raw (untransformed) BMI")

p2 <- ggplot(nyfs1, aes(x = 1/bmi)) +
  geom_histogram(aes(y = ..density..),
                 binwidth=0.005, fill = "darkcyan", color = "white") +
  stat_function(fun = dnorm,
                args = list(mean = mean(1/nyfs1$bmi),
                           sd = sd(1/nyfs1$bmi))) +
  labs(x = "1 / Body-Mass Index",
       title = "Inverse of BMI (1/BMI)")

gridExtra::grid.arrange(p1, p2, ncol=2,
top = textGrob("Comparing BMI to 1/BMI", gp=gpar(fontsize=15)))
```



```
# this approach to top label lets us adjust the size of type used
# in the main title
# note that you'll need to have called library(grid) or
# require(grid) for this to work properly
rm(p1, p2) # cleanup
```

When we are confronted with a variable that is not Normally distributed but that we wish was Normally distributed, it is sometimes useful to consider whether working with a **transformation** of the data will yield a more helpful result. The next Section provides some initial guidance about choosing between a class of power transformations that can reduce the impact of non-Normality in unimodal data.



# Chapter 9

## Using Transformations to “Normalize” Distributions

- When we are confronted with a variable that is not Normally distributed but that we wish was Normally distributed, it is sometimes useful to consider whether working with a transformation of the data will yield a more helpful result.
- Many statistical methods, including t tests and analyses of variance, assume Normal distributions.
- We'll discuss using R to assess a range of what are called Box-Cox power transformations, via plots, mainly.

### 9.1 The Ladder of Power Transformations

The key notion in re-expression of a single variable to obtain a distribution better approximated by the Normal or re-expression of an outcome in a simple regression model is that of a **ladder of power transformations**, which applies to any unimodal data.

Power	Transformation
3	$x^3$
2	$x^2$
1	x (unchanged)
0.5	$x^{0.5} = \sqrt{x}$
0	$\ln x$
-0.5	$x^{-0.5} = 1/\sqrt{x}$
-1	$x^{-1} = 1/x$
-2	$x^{-2} = 1/x^2$

### 9.2 Using the Ladder

As we move further away from the *identity* function (power = 1) we change the shape more and more in the same general direction.

- For instance, if we try a logarithm, and this seems like too much of a change, we might try a square root instead.
- Note that this ladder (which like many other things is due to John Tukey) uses the logarithm for the

“power zero” transformation rather than the constant, which is what  $x^0$  actually is.

- If the variable  $x$  can take on negative values, we might take a different approach. If  $x$  is a count of something that could be zero, we often simply add 1 to  $x$  before transformation.

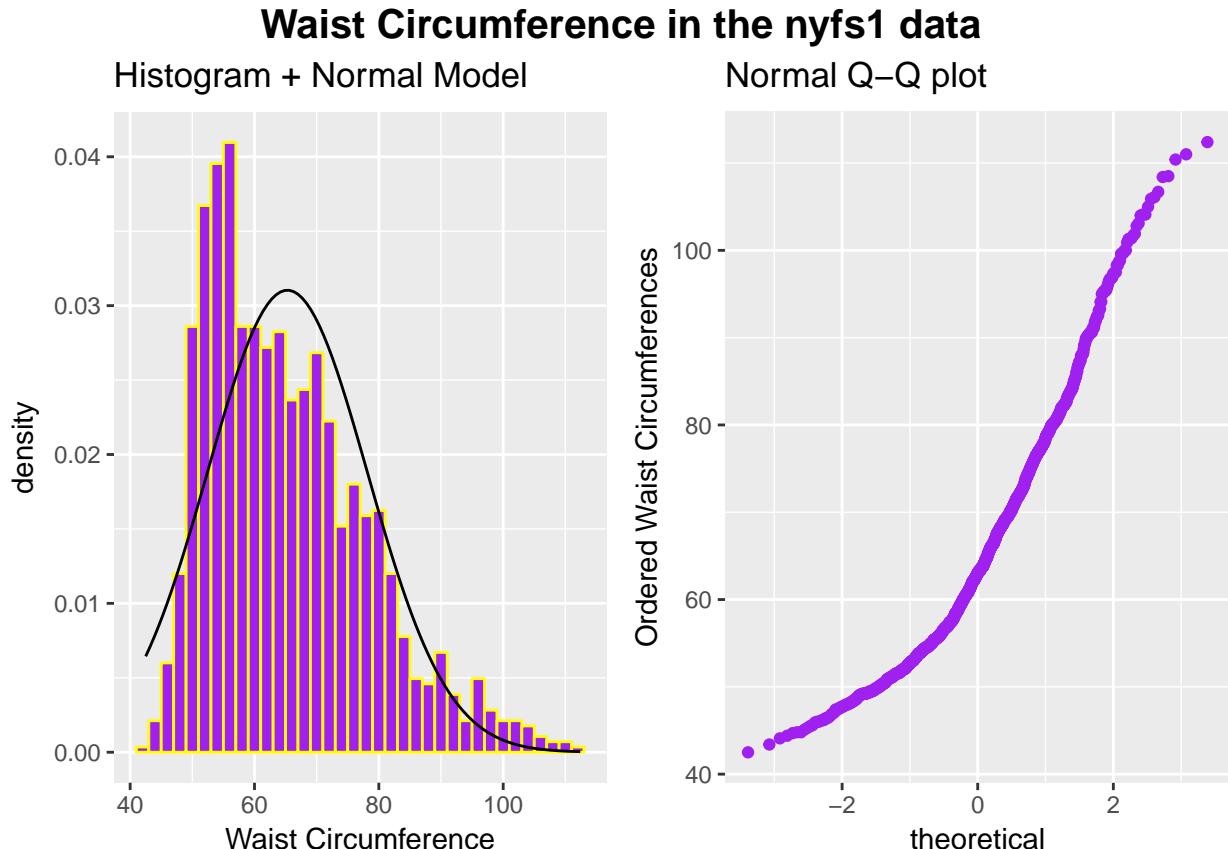
### 9.3 Can we transform Waist Circumferences?

Here are a pair of plots describing the waist circumference data in the NYFS data.

```
p1 <- ggplot(nyfs1, aes(x = waist.circ)) +
  geom_histogram(aes(y = ..density..),
                 binwidth=2, fill = "purple", color = "yellow") +
  stat_function(fun = dnorm, args = list(mean = mean(nyfs1$waist.circ),
                                         sd = sd(nyfs1$waist.circ))) +
  labs(x = "Waist Circumference", title="Histogram + Normal Model")

p2 <- ggplot(nyfs1, aes(sample = waist.circ)) +
  geom_point(stat="qq", color = "purple") +
  labs(y = "Ordered Waist Circumferences", title="Normal Q-Q plot")

library(grid)
# this approach to top label lets us adjust
# the size and font (here bold) used in the main title
gridExtra::grid.arrange(p1, p2, ncol=2,
                       top = textGrob("Waist Circumference in the nyfs1 data",
                                      gp=gpar(fontsize=15,font=2)))
```



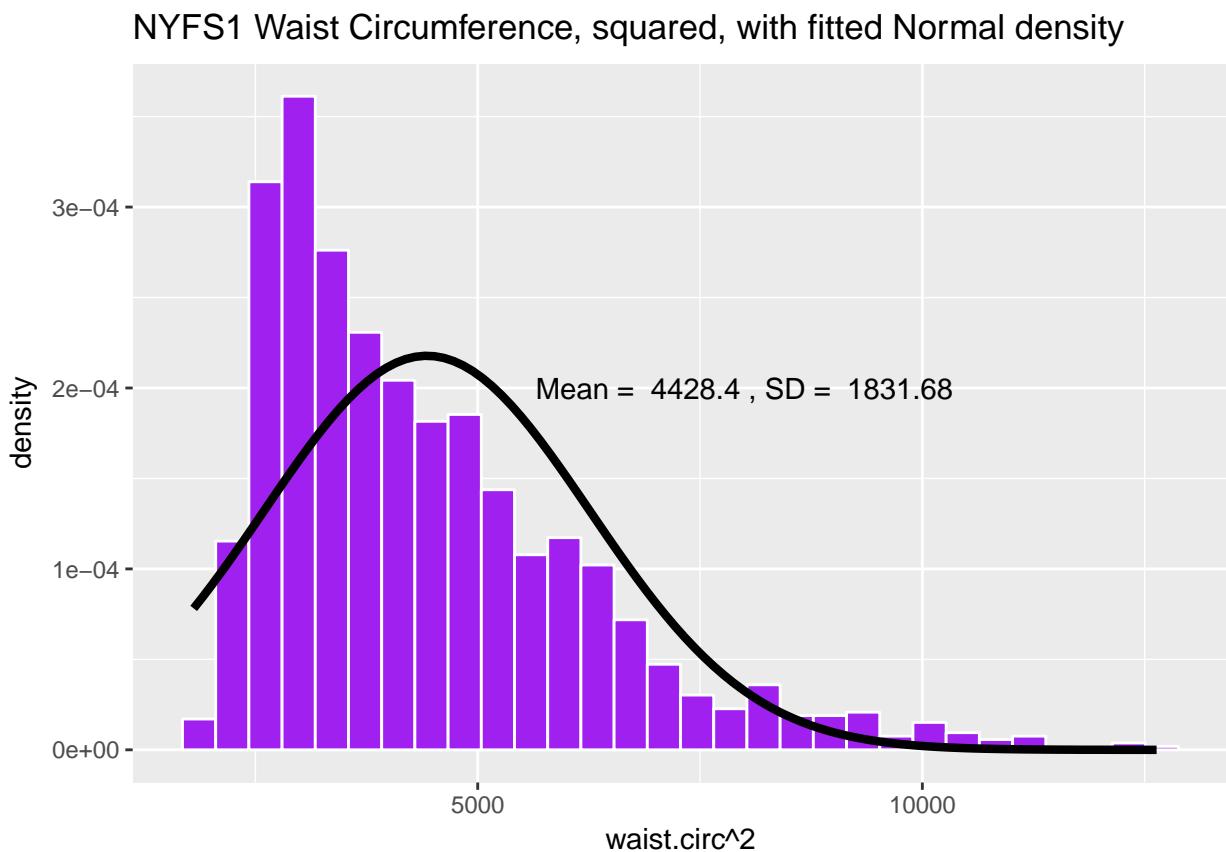
```
## clean up
rm(p1, p2)
```

All of the values are positive, naturally, and there is some sign of skew. If we want to use the tools of the Normal distribution to describe these data, we might try taking a step “up” our ladder from power 1 to power 2.

### 9.3.1 The Square

Does squaring the Waist Circumference data help to “Normalize” the histogram?

```
ggplot(nyfs1, aes(x = waist.circ^2)) +
  geom_histogram(aes(y = ..density..), bins = 30, fill = "purple", col="white") +
  stat_function(fun = dnorm, lwd = 1.5, col = "black",
                args = list(mean = mean(nyfs1$waist.circ^2), sd = sd(nyfs1$waist.circ^2))) +
  annotate("text", x = 8000, y = 0.0002, col = "black",
           label = paste("Mean = ", round(mean(nyfs1$waist.circ^2),2),
                         ", SD = ", round(sd(nyfs1$waist.circ^2),2))) +
  labs(title = "NYFS1 Waist Circumference, squared, with fitted Normal density")
```

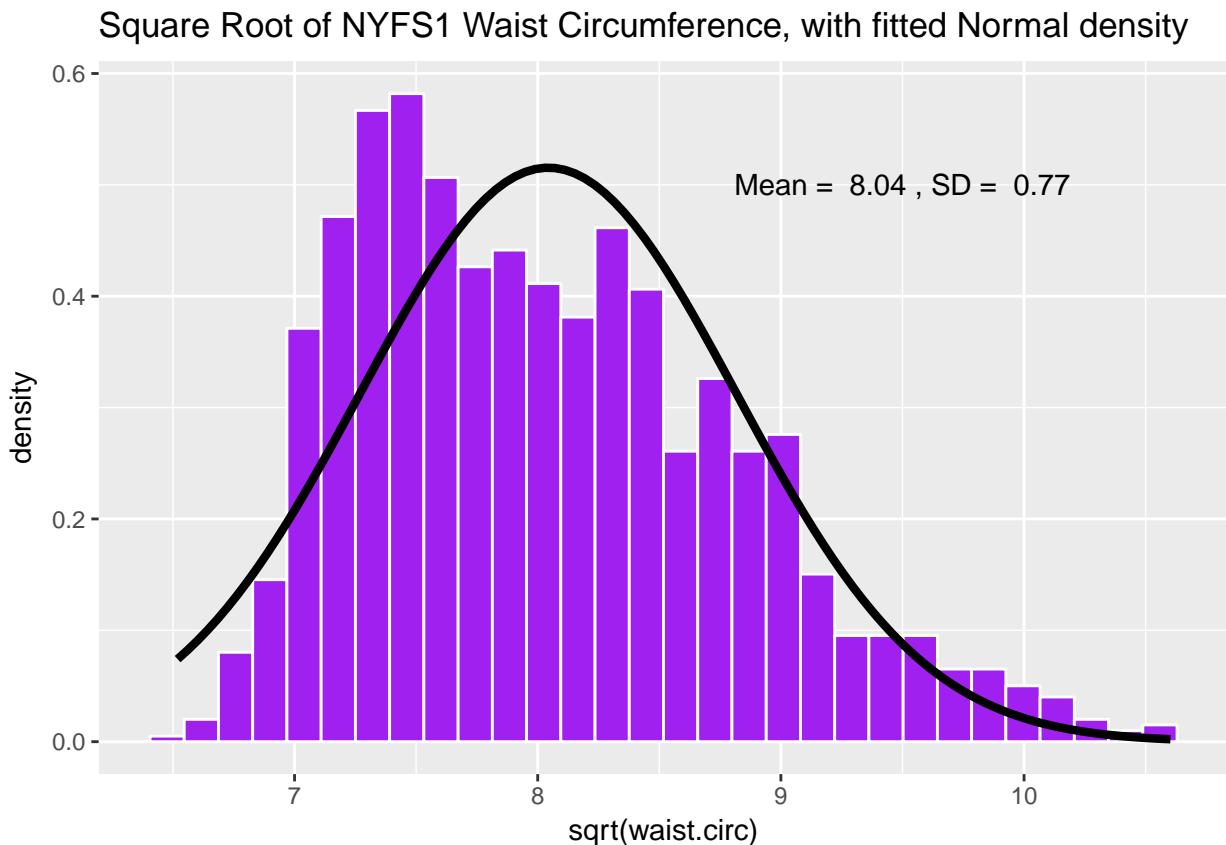


Looks like that was the wrong direction. Shall we try moving down the ladder instead?

### 9.3.2 The Square Root

Would a square root applied to the waist circumference data help alleviate that right skew?

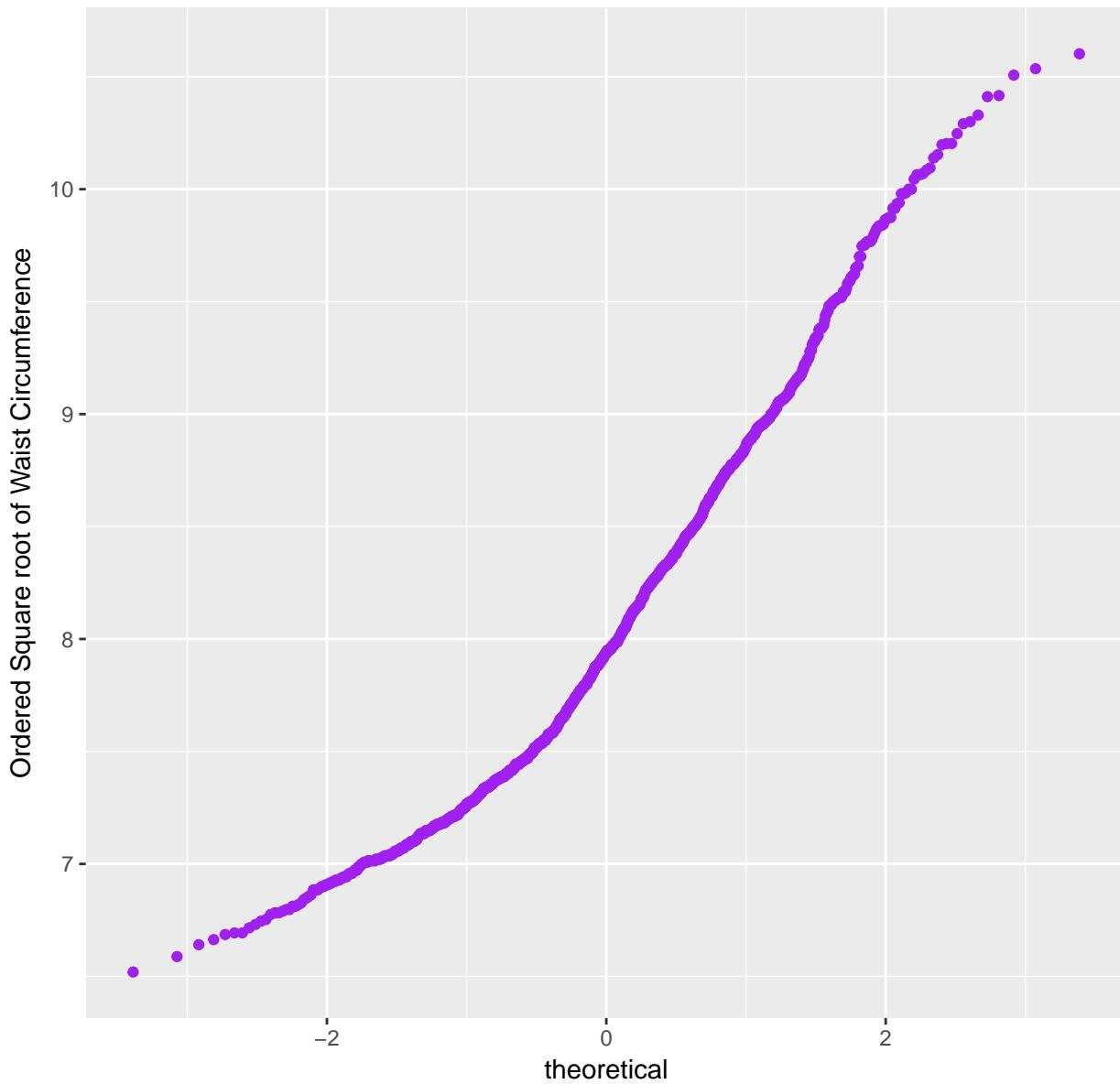
```
ggplot(nyfs1, aes(x = sqrt(waist.circ))) +
  geom_histogram(aes(y = ..density..), bins = 30,
                 fill = "purple", col="white") +
  stat_function(fun = dnorm, lwd = 1.5, col = "black",
                args = list(mean = mean(sqrt(nyfs1$waist.circ)),
                            sd = sd(sqrt(nyfs1$waist.circ)))) +
  annotate("text", x = 9.5, y = 0.5, col = "black",
           label = paste("Mean = ", round(mean(sqrt(nyfs1$waist.circ)),2),
                         ", SD = ", round(sd(sqrt(nyfs1$waist.circ)),2))) +
  labs(title = "Square Root of NYFS1 Waist Circumference, with fitted Normal density")
```



That looks a lot closer to a Normal distribution. Consider the Normal Q-Q plot below.

```
ggplot(nyfs1, aes(sample = sqrt(waist.circ))) +
  geom_point(stat="qq", color = "purple") +
  labs(y = "Ordered Square root of Waist Circumference",
       title="Normal Q-Q plot of Square Root of Waist Circumference")
```

Normal Q–Q plot of Square Root of Waist Circumference



### 9.3.3 The Logarithm

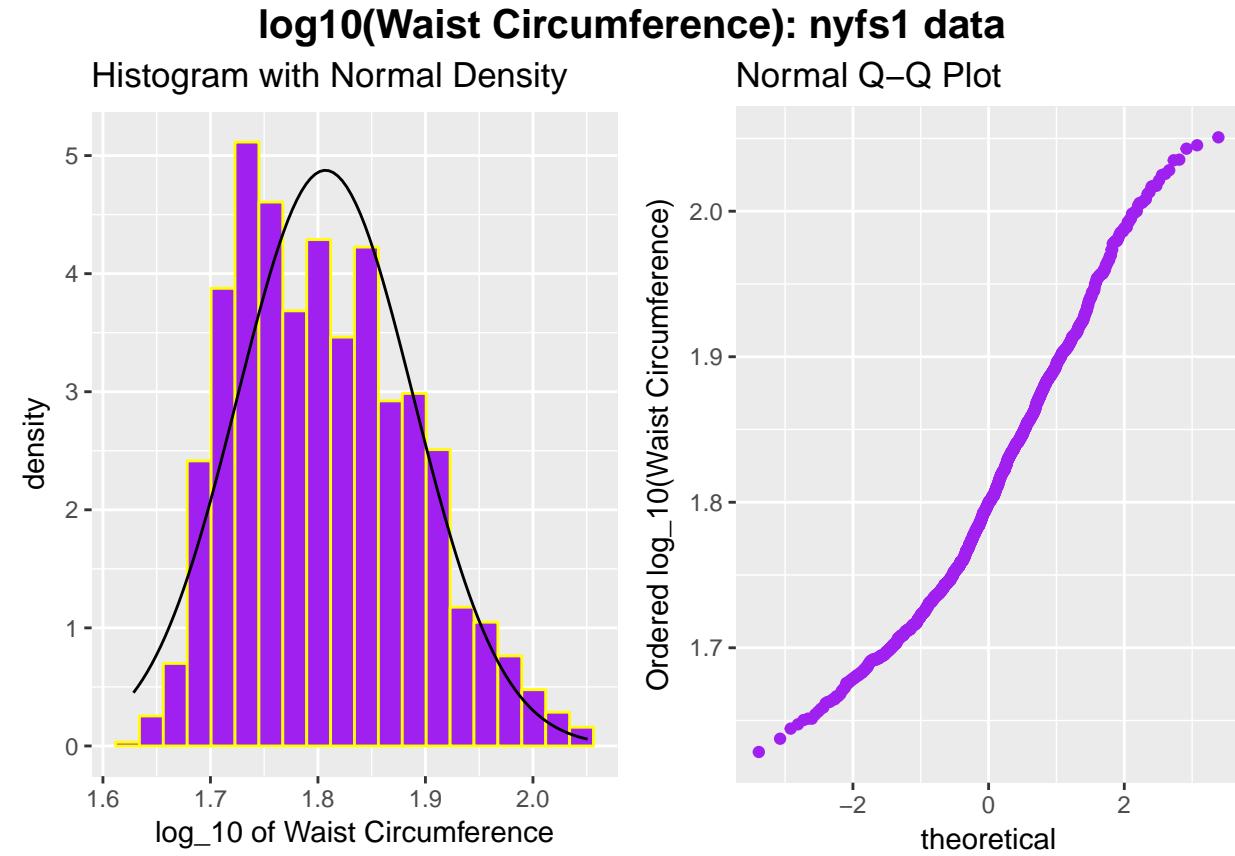
We might also try a logarithm of the waist circumference data. We can use either the natural logarithm (`log`, in R) or the base-10 logarithm (`log10`, in R) - either will have the same impact on skew.

```
p1 <- ggplot(nyfs1, aes(x = log10(waist.circ))) +
  geom_histogram(aes(y = ..density..),
                 bins=20, fill = "purple", color = "yellow") +
  stat_function(fun = dnorm, args = list(mean = mean(log10(nyfs1$waist.circ)),
                                         sd = sd(log10(nyfs1$waist.circ)))) +
  labs(x = "log10 of Waist Circumference", title="Histogram with Normal Density")

p2 <- ggplot(nyfs1, aes(sample = log10(waist.circ))) +
```

```
geom_point(stat="qq", color = "purple") +
  labs(y = "Ordered log_10(Waist Circumference)", title="Normal Q-Q Plot")

gridExtra::grid.arrange(p1, p2, ncol=2,
  top = textGrob("log10(Waist Circumference): nyfs1 data",
    gp=gpar(fontsize=15,font=2)))
```

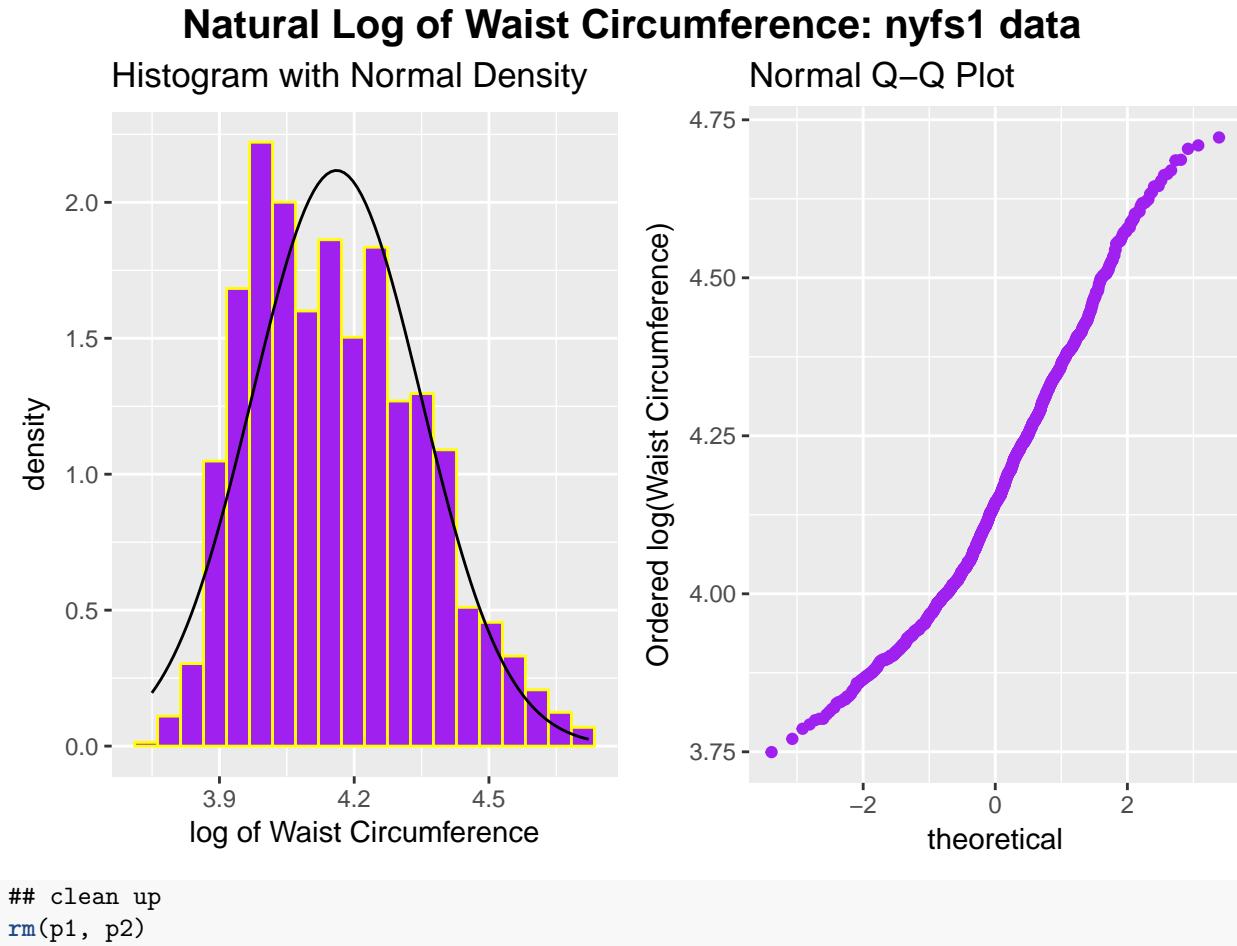


```
## clean up
rm(p1, p2)

p1 <- ggplot(nyfs1, aes(x = log(waist.circ))) +
  geom_histogram(aes(y = ..density..),
    bins=20, fill = "purple", color = "yellow") +
  stat_function(fun = dnorm,
    args = list(mean = mean(log(nyfs1$waist.circ)),
      sd = sd(log(nyfs1$waist.circ)))) +
  labs(x = "log of Waist Circumference", title="Histogram with Normal Density")

p2 <- ggplot(nyfs1, aes(sample = log(waist.circ))) +
  geom_point(stat="qq", color = "purple") +
  labs(y = "Ordered log(Waist Circumference)", title="Normal Q–Q Plot")

gridExtra::grid.arrange(p1, p2, ncol=2,
  top = textGrob("Natural Log of Waist Circumference: nyfs1 data",
    gp=gpar(fontsize=15,font=2)))
```

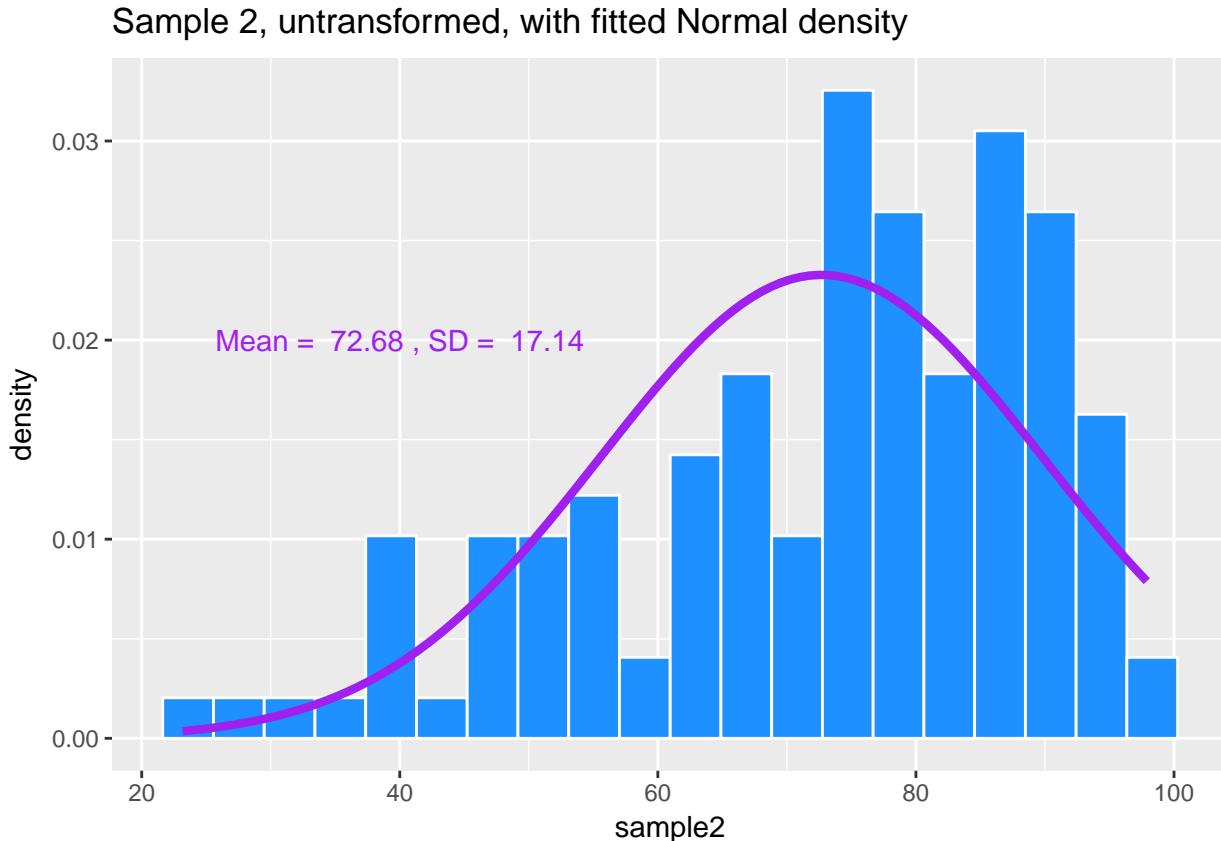


## 9.4 A Simulated Data Set with Left Skew

```
set.seed(431); data2 <- data.frame(sample2 = 100*rbeta(n = 125, shape1 = 5, shape2 = 2))
```

If we'd like to transform these data so as to better approximate a Normal distribution, where should we start? What transformation do you suggest?

```
ggplot(data2, aes(x = sample2)) +
  geom_histogram(aes(y = ..density..),
                 bins = 20, fill = "dodgerblue", col="white") +
  stat_function(fun = dnorm, lwd = 1.5, col = "purple",
                args = list(mean = mean(data2$sample2),
                            sd = sd(data2$sample2))) +
  annotate("text", x = 40, y = 0.02, col = "purple",
           label = paste("Mean = ", round(mean(data2$sample2),2),
                         ", SD = ", round(sd(data2$sample2),2))) +
  labs(title = "Sample 2, untransformed, with fitted Normal density")
```



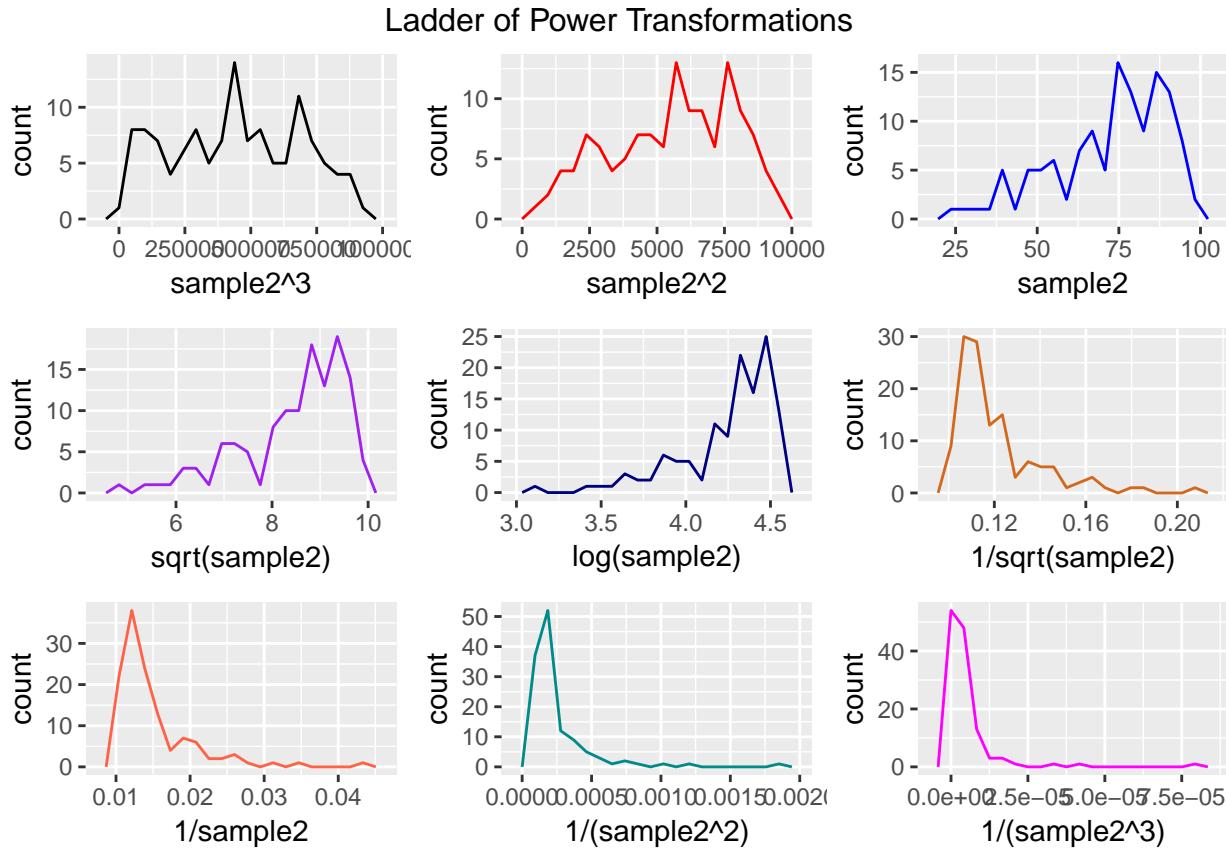
## 9.5 Transformation Example 2: Ladder of Potential Transformations in Frequency Polygons

```

p1 <- ggplot(data2, aes(x = sample2^3)) + geom_freqpoly(col = "black", bins = 20)
p2 <- ggplot(data2, aes(x = sample2^2)) + geom_freqpoly(col = "red", bins = 20)
p3 <- ggplot(data2, aes(x = sample2)) + geom_freqpoly(col = "blue", bins = 20)
p4 <- ggplot(data2, aes(x = sqrt(sample2))) + geom_freqpoly(col = "purple", bins = 20)
p5 <- ggplot(data2, aes(x = log(sample2))) + geom_freqpoly(col = "navy", bins = 20)
p6 <- ggplot(data2, aes(x = 1/sqrt(sample2))) + geom_freqpoly(col = "chocolate", bins = 20)
p7 <- ggplot(data2, aes(x = 1/sample2)) + geom_freqpoly(col = "tomato", bins = 20)
p8 <- ggplot(data2, aes(x = 1/(sample2^2))) + geom_freqpoly(col = "darkcyan", bins = 20)
p9 <- ggplot(data2, aes(x = 1/(sample2^3))) + geom_freqpoly(col = "magenta", bins = 20)

gridExtra::grid.arrange(p1, p2, p3, p4, p5, p6, p7, p8, p9, nrow=3,
top="Ladder of Power Transformations")

```



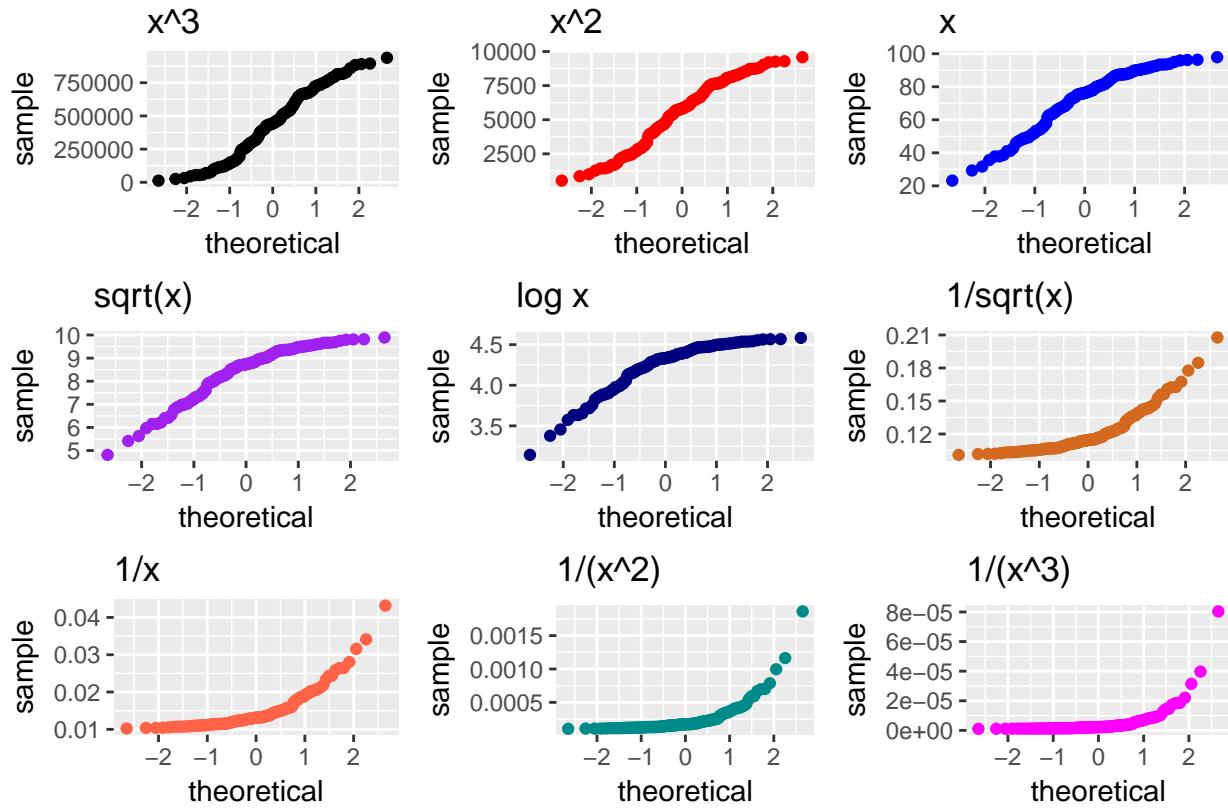
## 9.6 Transformation Example 2 Ladder with Normal Q-Q Plots

```

p1 <- ggplot(data2, aes(sample = sample2^3)) +
  geom_point(stat="qq", col = "black") + labs(title = "x^3")
p2 <- ggplot(data2, aes(sample = sample2^2)) +
  geom_point(stat="qq", col = "red") + labs(title = "x^2")
p3 <- ggplot(data2, aes(sample = sample2)) +
  geom_point(stat="qq", col = "blue") + labs(title = "x")
p4 <- ggplot(data2, aes(sample = sqrt(sample2))) +
  geom_point(stat="qq", col = "purple") + labs(title = "sqrt(x)")
p5 <- ggplot(data2, aes(sample = log(sample2))) +
  geom_point(stat="qq", col = "navy") + labs(title = "log x")
p6 <- ggplot(data2, aes(sample = 1/sqrt(sample2))) +
  geom_point(stat="qq", col = "chocolate") + labs(title = "1/sqrt(x)")
p7 <- ggplot(data2, aes(sample = 1/sample2)) +
  geom_point(stat="qq", col = "tomato") + labs(title = "1/x")
p8 <- ggplot(data2, aes(sample = 1/(sample2^2))) +
  geom_point(stat="qq", col = "darkcyan") + labs(title = "1/(x^2)")
p9 <- ggplot(data2, aes(sample = 1/(sample2^3))) +
  geom_point(stat="qq", col = "magenta") + labs(title = "1/(x^3)")

gridExtra::grid.arrange(p1, p2, p3, p4, p5, p6, p7, p8, p9, nrow=3,
bottom="Ladder of Power Transformations")

```



Ladder of Power Transformations

It looks like taking the square of the data produces the most “Normalish” plot in this case.

# Chapter 10

## Summarizing data within subgroups

### 10.1 Using dplyr and summarise to build a tibble of summary information

```
nyfs1 %>%
  group_by(sex) %>%
  select(bmi, waist.circ, sex) %>%
  summarise_all(funs(median))

# A tibble: 2 x 3
  sex     bmi waist.circ
  <fctr> <dbl>      <dbl>
1 Female   17.6      63.6
2 Male     17.7      62.5

nyfs1 %>%
  group_by(bmi.cat) %>%
  summarise(mean = mean(waist.circ), sd = sd(waist.circ), median = median(waist.circ),
            skew_1 = round((mean(waist.circ) - median(waist.circ)) / sd(waist.circ),3))

# A tibble: 4 x 5
  bmi.cat    mean     sd median skew_1
  <fctr> <dbl> <dbl>  <dbl>  <dbl>
1 1 Underweight 54.9  7.63  53.9  0.136
2 2 Normal weight 61.0  9.10  59.2  0.193
3 3 Overweight  71.1 11.80  72.0 -0.075
4 4 Obese       79.9 15.01  79.9 -0.003
```

While patients in the heavier groups generally had higher waist circumferences, this is not inevitably the case.

The data transformation with dplyr cheat sheet found under the Help menu in R Studio is a great resource. And, of course, for more details, visit Grolemund and Wickham (2017).

### 10.2 Using the by function to summarize groups numerically

We can summarize our data numerically in multiple ways, but to use the `favstats` or `Hmisc::describe` tools to each individual BMI subgroup separately, we might consider applying the `by` function.

```
by(nyfs1$waist.circ, nyfs1$bmi.cat, mosaic::favstats)
```

```
nyfs1$bmi.cat: 1 Underweight
  min   Q1 median   Q3 max mean   sd n missing
 42.5 49.2   53.9 62.4 68.5 54.9 7.63 42      0
-----
nyfs1$bmi.cat: 2 Normal weight
  min   Q1 median   Q3 max mean   sd n missing
 44.1 53.8   59.2 68 85.5   61 9.1 926      0
-----
nyfs1$bmi.cat: 3 Overweight
  min   Q1 median   Q3 max mean   sd n missing
 49.3 60.8   72 80.6 98.3 71.1 11.8 237      0
-----
nyfs1$bmi.cat: 4 Obese
  min   Q1 median   Q3 max mean   sd n missing
 52.1 66.7   79.9 91.6 112 79.9 15 211      0
```

As shown below, we could do this in pieces with `dplyr`, but the `by` approach can be faster for this sort of thing.

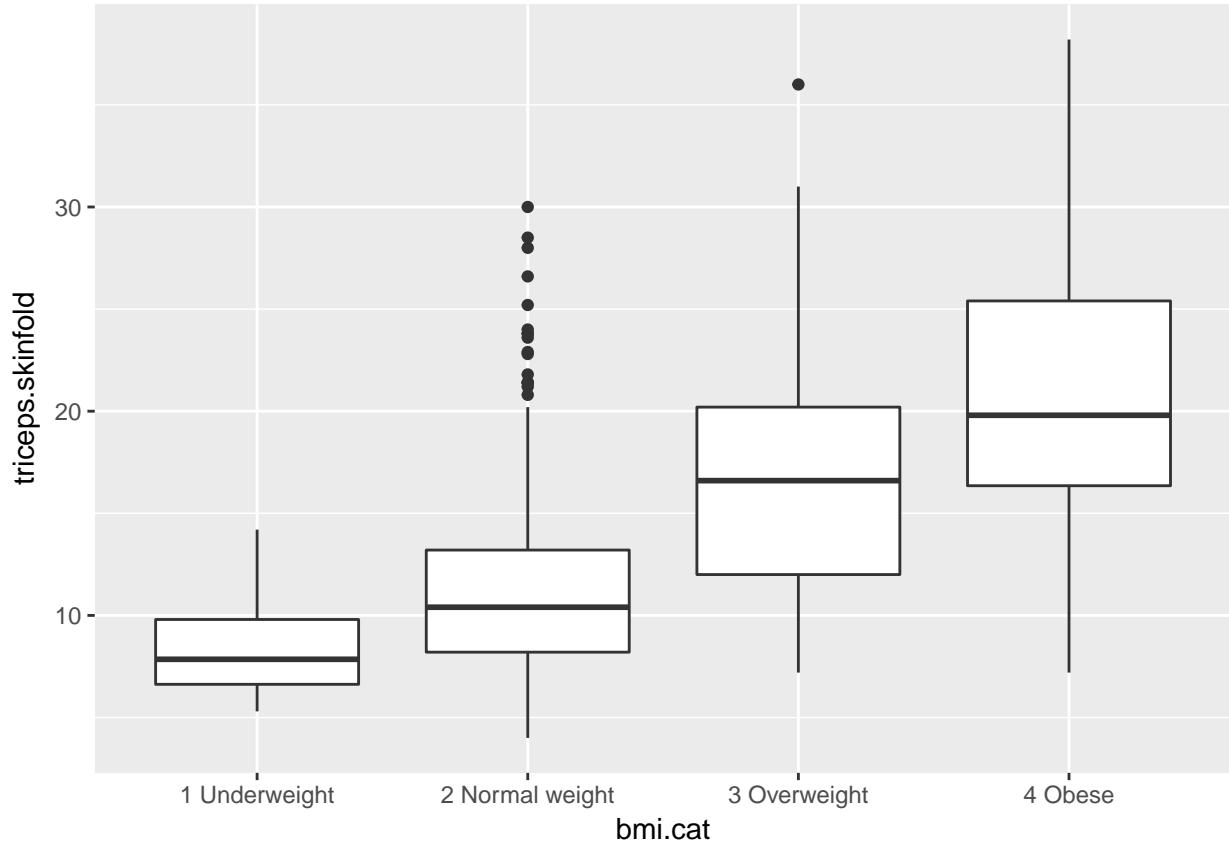
```
nyfs1 %>%
  group_by(bmi.cat) %>%
  summarise(min = min(waist.circ), Q1 = quantile(waist.circ, 0.25),
            median = median(waist.circ), Q3 = quantile(waist.circ, 0.75),
            max = max(waist.circ), mean = mean(waist.circ),
            sd = sd(waist.circ), n = length(waist.circ),
            missing = sum(is.na(waist.circ)))
```

```
# A tibble: 4 x 10
  bmi.cat   min   Q1 median   Q3 max mean   sd n missing
  <fctr> <dbl> <dbl> <dbl> <dbl> <dbl> <dbl> <int> <int>
1 1 Underweight 42.5 49.2   53.9 62.4 68.5 54.9 7.63 42      0
2 2 Normal weight 44.1 53.8   59.2 68.0 85.5 61.0 9.10 926      0
3 3 Overweight 49.3 60.8   72.0 80.6 98.3 71.1 11.80 237      0
4 4 Obese     52.1 66.7   79.9 91.6 112.4 79.9 15.01 211      0
```

## 10.3 Boxplots to Relate an Outcome to a Categorical Predictor

Boxplots are much more useful when comparing samples of data. For instance, consider this comparison boxplot describing the triceps skinfold results across the four levels of BMI category.

```
ggplot(nyfs1, aes(x=bmi.cat, y=triceps.skinfold)) +
  geom_boxplot()
```

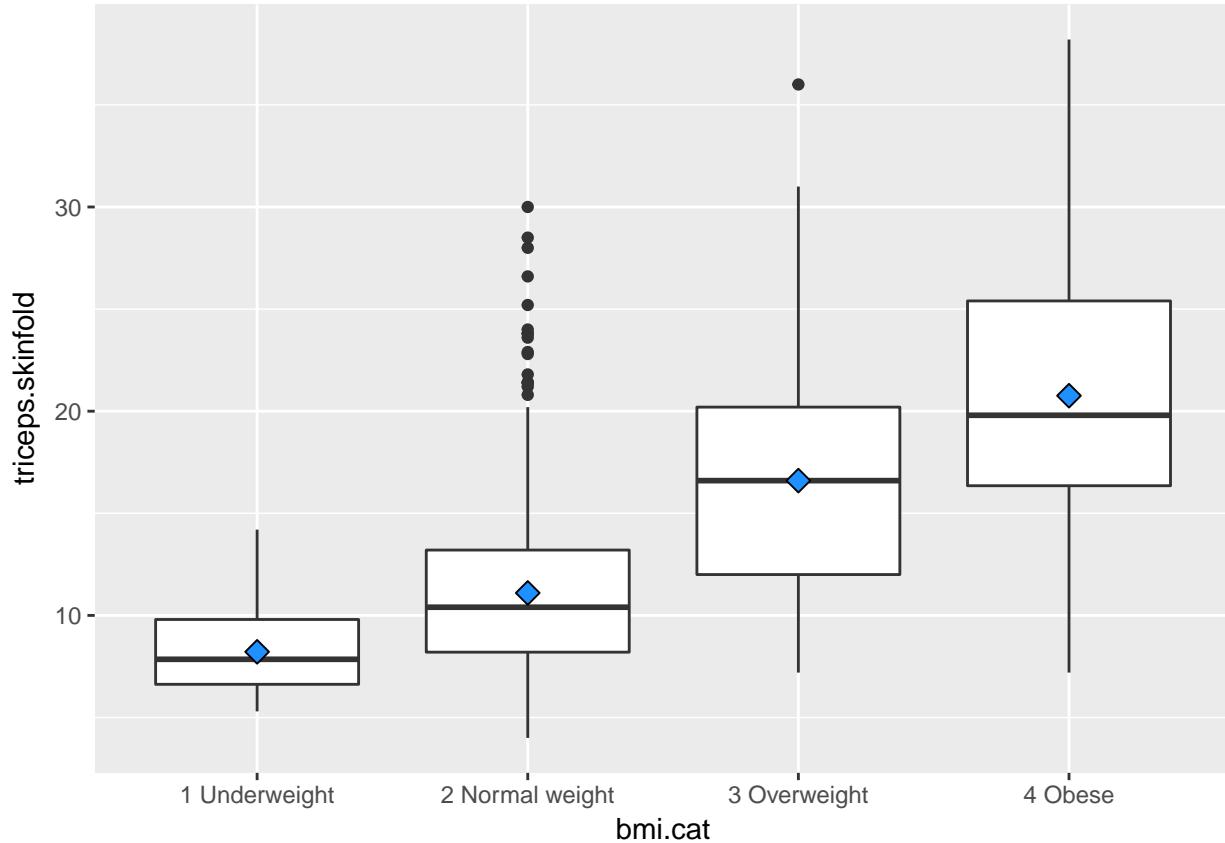


As always, the boxplot shows the five-number summary (minimum, 25th percentile, median, 75th percentile and maximum) in addition to highlighting candidate outliers.

### 10.3.1 Augmenting the Boxplot with the Sample Mean

Often, we want to augment such a plot, perhaps with the **sample mean** within each category, so as to highlight skew (in terms of whether the mean is meaningfully different from the median.)

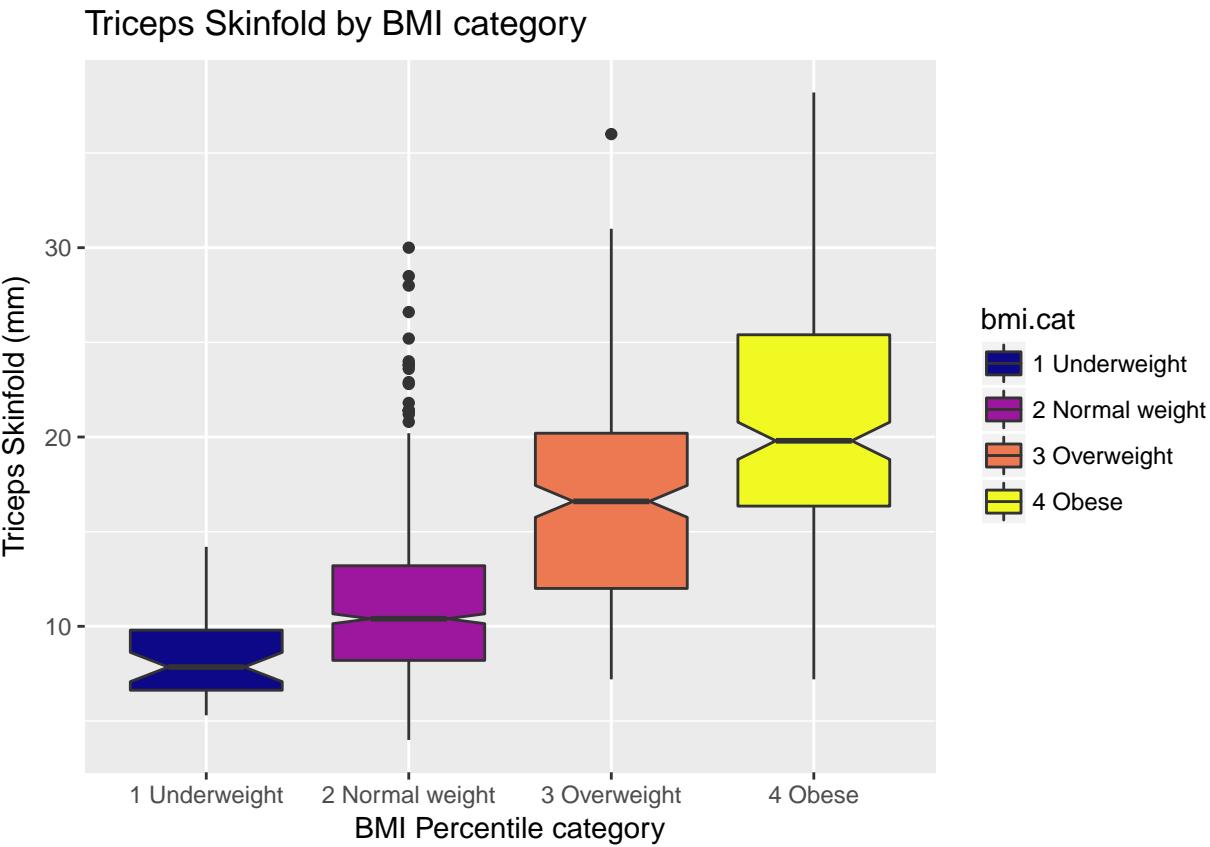
```
ggplot(nyfs1, aes(x=bmi.cat, y=triceps.skinfold)) +
  geom_boxplot() +
  stat_summary(fun.y="mean", geom="point", shape=23, size=3, fill="dodgerblue")
```



### 10.3.2 Adding Notches to a Boxplot

**Notches** are used in boxplots to help visually assess whether the medians of the distributions across the various groups actually differ to a statistically detectable extent. Think of them as confidence regions around the medians. If the notches do not overlap, as in this situation, this provides some evidence that the medians in the populations represented by these samples may be different.

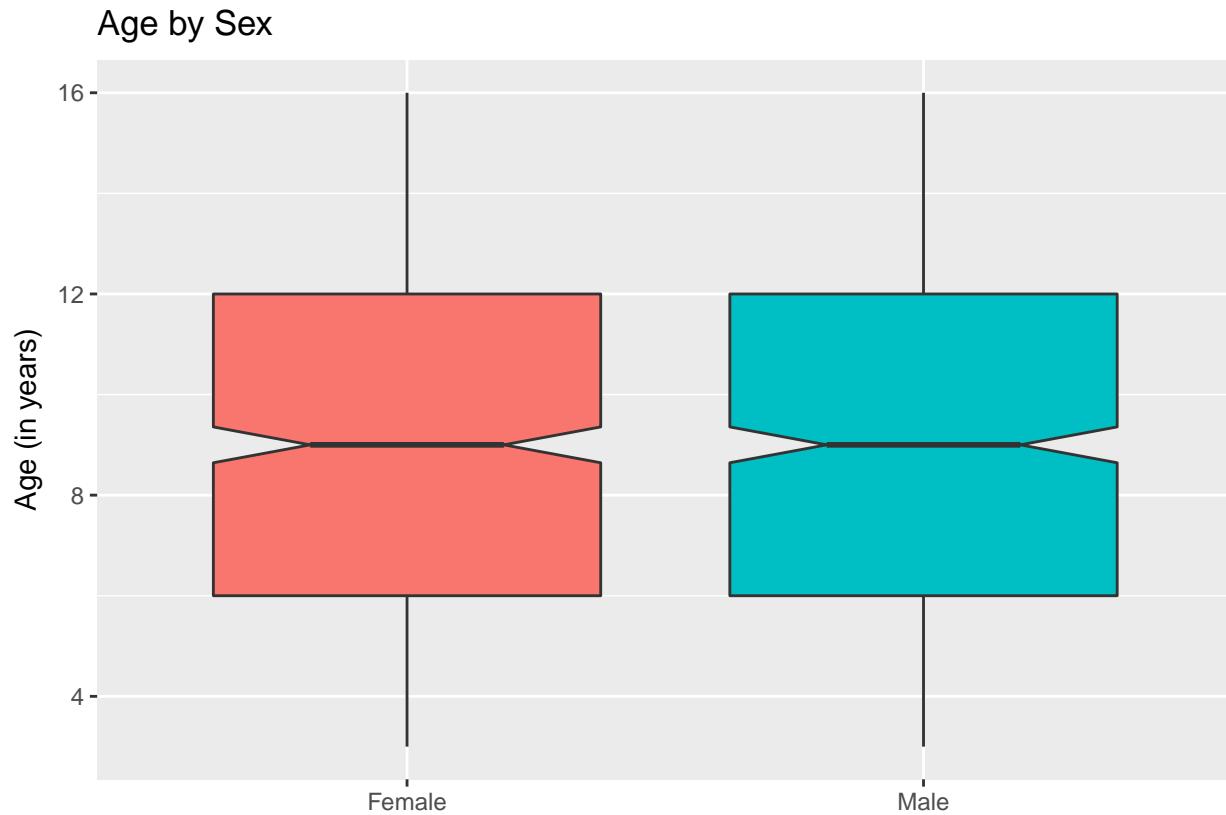
```
ggplot(nyfs1, aes(x=bmi.cat, y=triceps.skinfold, fill = bmi.cat)) +
  geom_boxplot(notch=TRUE) +
  scale_fill_viridis(discrete=TRUE, option="plasma") +
  labs(title = "Triceps Skinfold by BMI category",
       x = "BMI Percentile category", y = "Triceps Skinfold (mm)")
```



There is no overlap between the notches for each of the four categories, so we might reasonably conclude that the true median triceps skinfold values across the four categories are statistically significantly different.

For an example where the notches overlap, consider the comparison of ages across sex.

```
ggplot(nyfs1, aes(x=sex, y=age.exam, fill=sex)) +
  geom_boxplot(notch=TRUE) +
  guides(fill = "none") ## drops the legend
  labs(title = "Age by Sex", x = "", y = "Age (in years)")
```



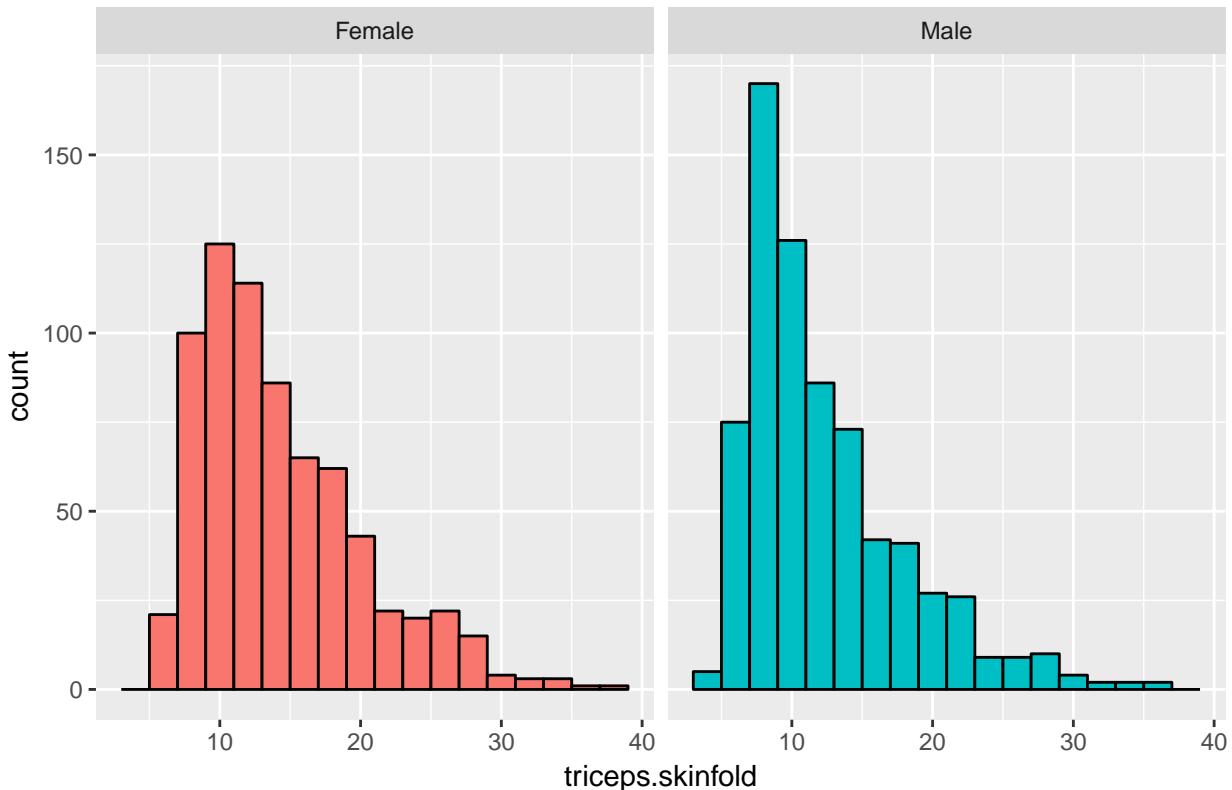
In this case, the overlap in the notches suggests that the median ages in the population of interest don't necessarily differ by sex.

## 10.4 Using Multiple Histograms to Make Comparisons

We can make an array of histograms to describe multiple groups of data, using `ggplot2` and the notion of **faceting** our plot.

```
ggplot(nyfs1, aes(x=triceps.skinfold, fill = sex)) +
  geom_histogram(binwidth = 2, color = "black") +
  facet_wrap(~ sex) +
  guides(fill = "none") +
  labs(title = "Triceps Skinfold by Sex")
```

### Triceps Skinfold by Sex

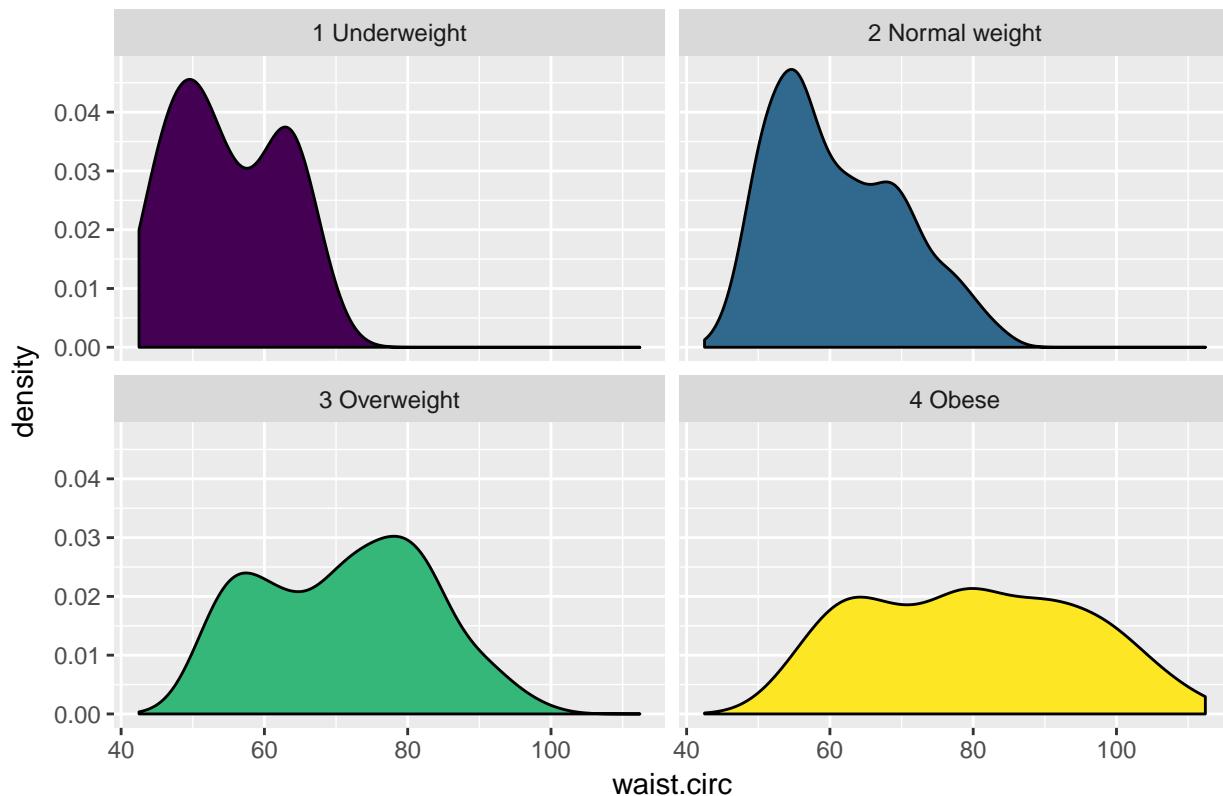


## 10.5 Using Multiple Density Plots to Make Comparisons

Or, we can make a series of density plots to describe multiple groups of data.

```
ggplot(nyfs1, aes(x=waist.circ, fill = bmi.cat)) +
  geom_density() +
  facet_wrap(~ bmi.cat) +
  scale_fill_viridis(discrete=T) +
  guides(fill = "none") +
  labs(title = "Waist Circumference by BMI Category")
```

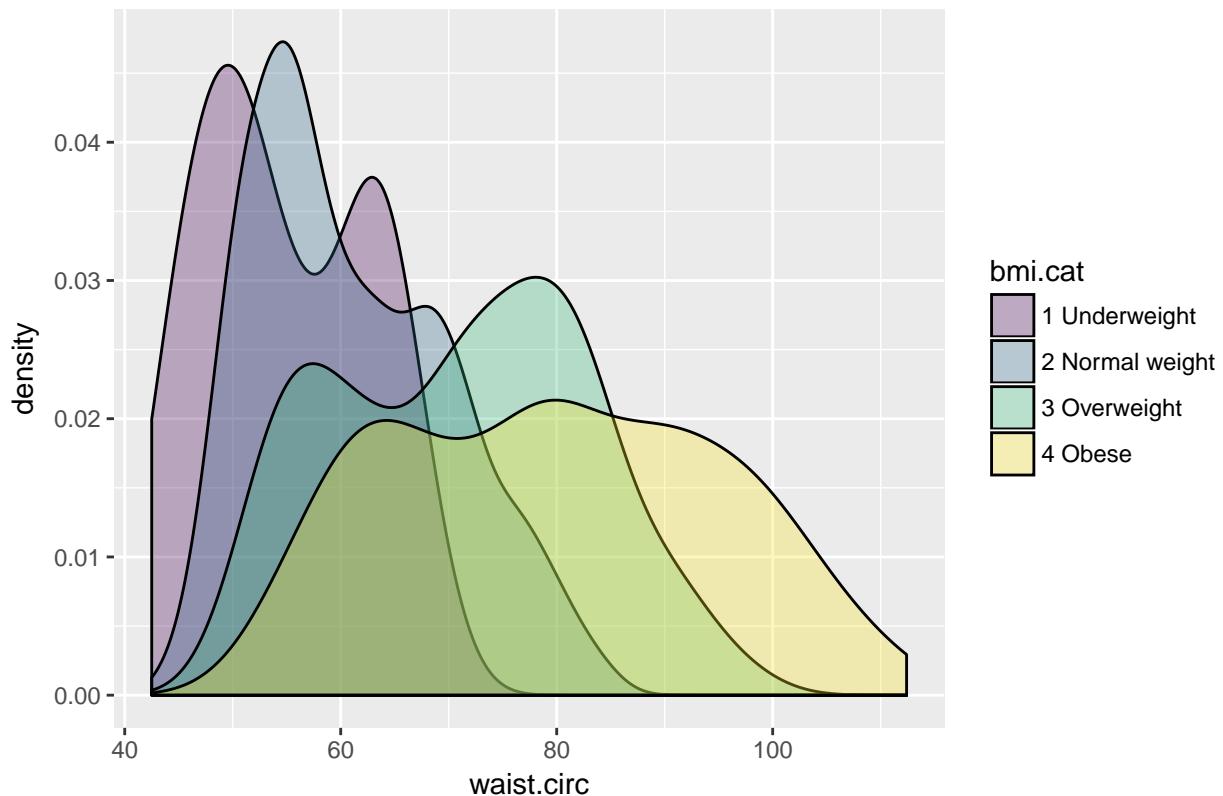
### Waist Circumference by BMI Category



Or, we can plot all of the densities on top of each other with semi-transparent fills.

```
ggplot(nyfs1, aes(x=waist.circ, fill=bmi.cat)) +
  geom_density(alpha=0.3) +
  scale_fill_viridis(discrete=T) +
  labs(title = "Waist Circumference by BMI Category")
```

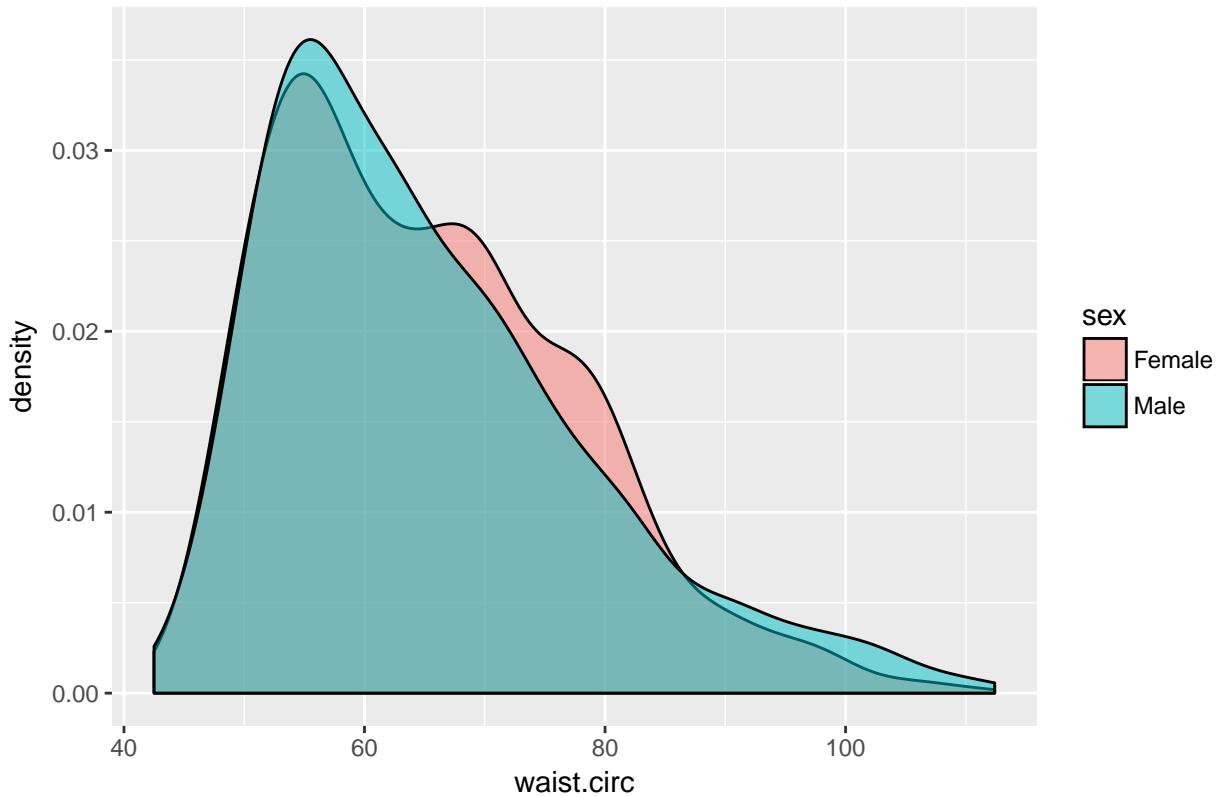
### Waist Circumference by BMI Category



This really works better when we are comparing only two groups, like females to males.

```
ggplot(nyfs1, aes(x=waist.circ, fill=sex)) +  
  geom_density(alpha=0.5) +  
  labs(title = "Waist Circumference by Sex")
```

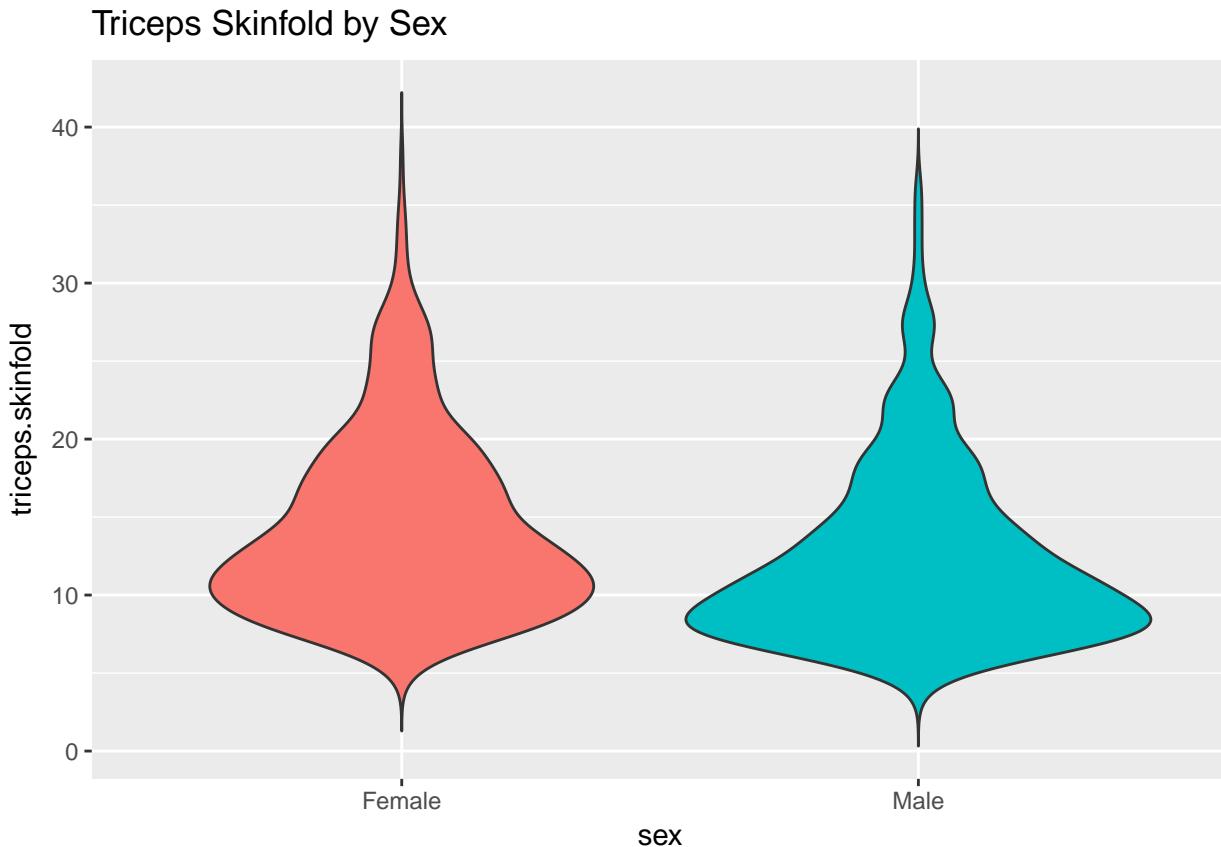
### Waist Circumference by Sex



## 10.6 Building a Violin Plot

There are a number of other plots which compare distributions of data sets. An interesting one is called a **violin plot**. A violin plot is a kernel density estimate, mirrored to form a symmetrical shape.

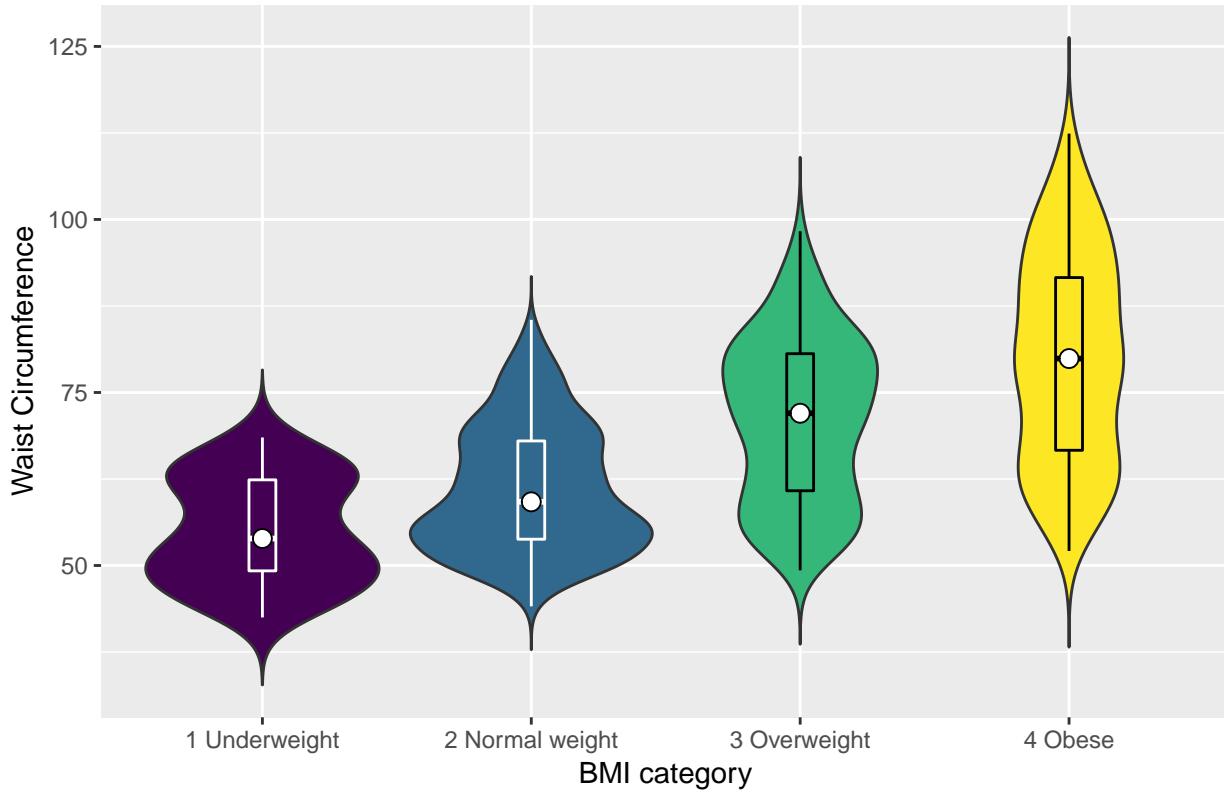
```
ggplot(nyfs1, aes(x=sex, y=triceps.skinfold, fill = sex)) +
  geom_violin(trim=FALSE) +
  guides(fill = "none") +
  labs(title = "Triceps Skinfold by Sex")
```



Traditionally, these plots are shown with overlaid boxplots and a white dot at the median, like this.

```
ggplot(nyfs1, aes(x=bmi.cat, y=waist.circ, fill = bmi.cat)) +
  geom_violin(trim=FALSE) +
  geom_boxplot(width=.1, outlier.colour=NA,
               color = c(rep("white",2), rep("black",2))) +
  stat_summary(fun.y=median, geom="point",
              fill="white", shape=21, size=3) +
  scale_fill_viridis(discrete=T) +
  guides(fill = "none") +
  labs(title = "Waist Circumference by BMI Category in nyfs1",
       x = "BMI category", y = "Waist Circumference")
```

### Waist Circumference by BMI Category in nyfs1



## 10.7 A Ridgeline Plot

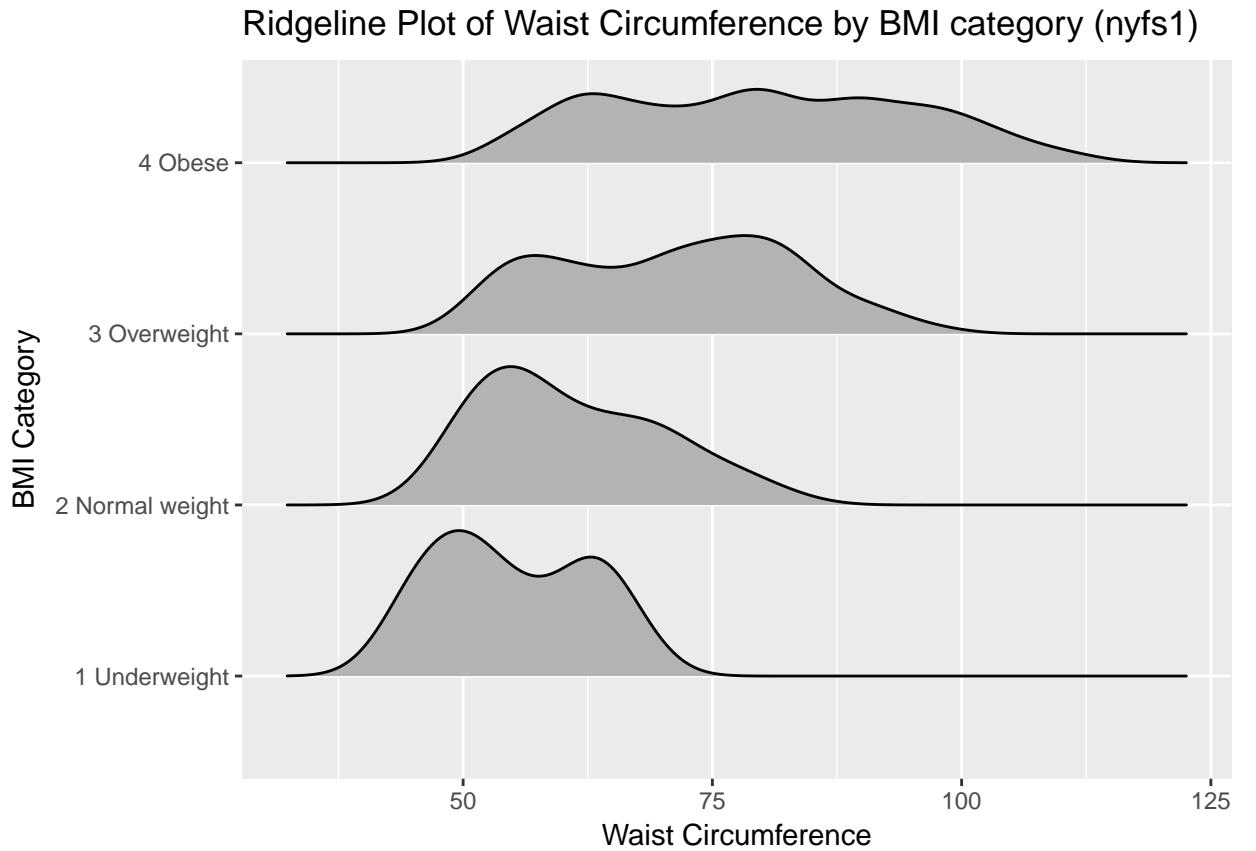
Some people don't like violin plots - for example, see <https://simplystatistics.org/2017/07/13/the-joy-of-no-more-violin-plots/>. A very new and attractive alternative plot is available. This shows the distribution of several groups simultaneously, especially when you have lots of subgroup categories, and is called a **ridgeline plot**<sup>1</sup>.

```
nyfs1 %>%
  ggplot(aes(x = waist.circ, y = bmi.cat, height = ..density..)) +
  ggridges::geom_density_ridges(scale = 0.85) +
  labs(title = "Ridgeline Plot of Waist Circumference by BMI category (nyfs1)",
       x = "Waist Circumference", y = "BMI Category")
```

Picking joint bandwidth of 3.38

---

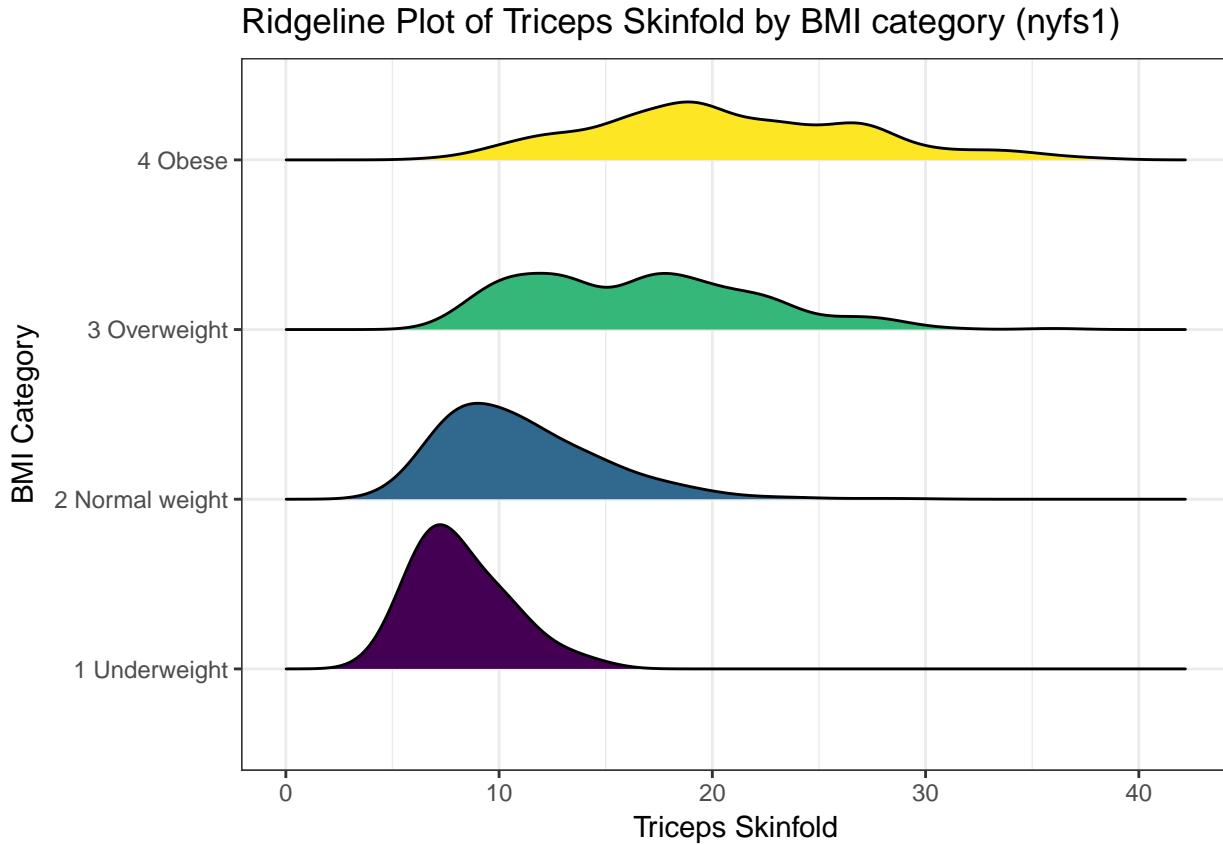
<sup>1</sup>These were originally called joy plots, and the tools were contained in the `ggjoy` package but that name and package has been deprecated in favor of `ggridges`.



And here's a ridgeline plot for the triceps skinfold. We'll start by sorting the subgroups by the median value of our outcome (triceps skinfold) in this case, though it turns out not to matter. We'll also add some color.

```
nyfs1 %>%
  mutate(bmi.cat = reorder(bmi.cat, triceps.skinfold, median)) %>%
  ggplot(aes(x = triceps.skinfold, y = bmi.cat, fill = bmi.cat, height = ..density..)) +
  ggridges::geom_density_ridges(scale = 0.85) +
  scale_fill_viridis(discrete = TRUE) +
  guides(fill = FALSE) +
  labs(title = "Ridgeline Plot of Triceps Skinfold by BMI category (nyfs1)",
       x = "Triceps Skinfold", y = "BMI Category") +
  theme_bw()
```

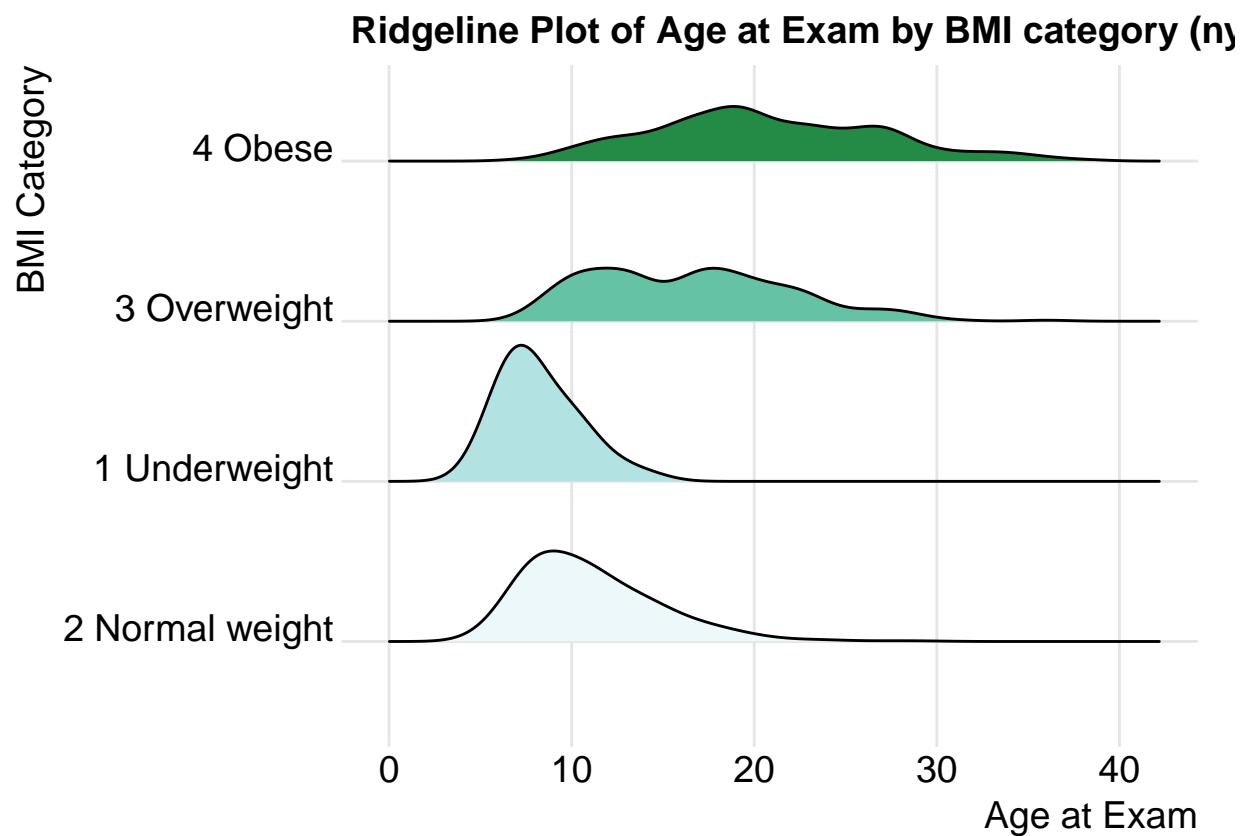
Picking joint bandwidth of 1.33



For one last example, we'll look at age by BMI category, so that sorting the BMI subgroups by the median matters, and we'll try an alternate color scheme, and a theme specially designed for the ridgeline plot.

```
nyfs1 %>%
  mutate(bmi.cat = reorder(bmi.cat, age.exam, median)) %>%
  ggplot(aes(x = triceps.skinfold, y = bmi.cat, fill = bmi.cat, height = ..density..)) +
  ggridges::geom_density_ridges(scale = 0.85) +
  scale_fill_brewer(palette = 2) +
  guides(fill = FALSE) +
  labs(title = "Ridgeline Plot of Age at Exam by BMI category (nyfs1)",
       x = "Age at Exam", y = "BMI Category") +
  ggridges::theme_ridges()
```

Picking joint bandwidth of 1.33





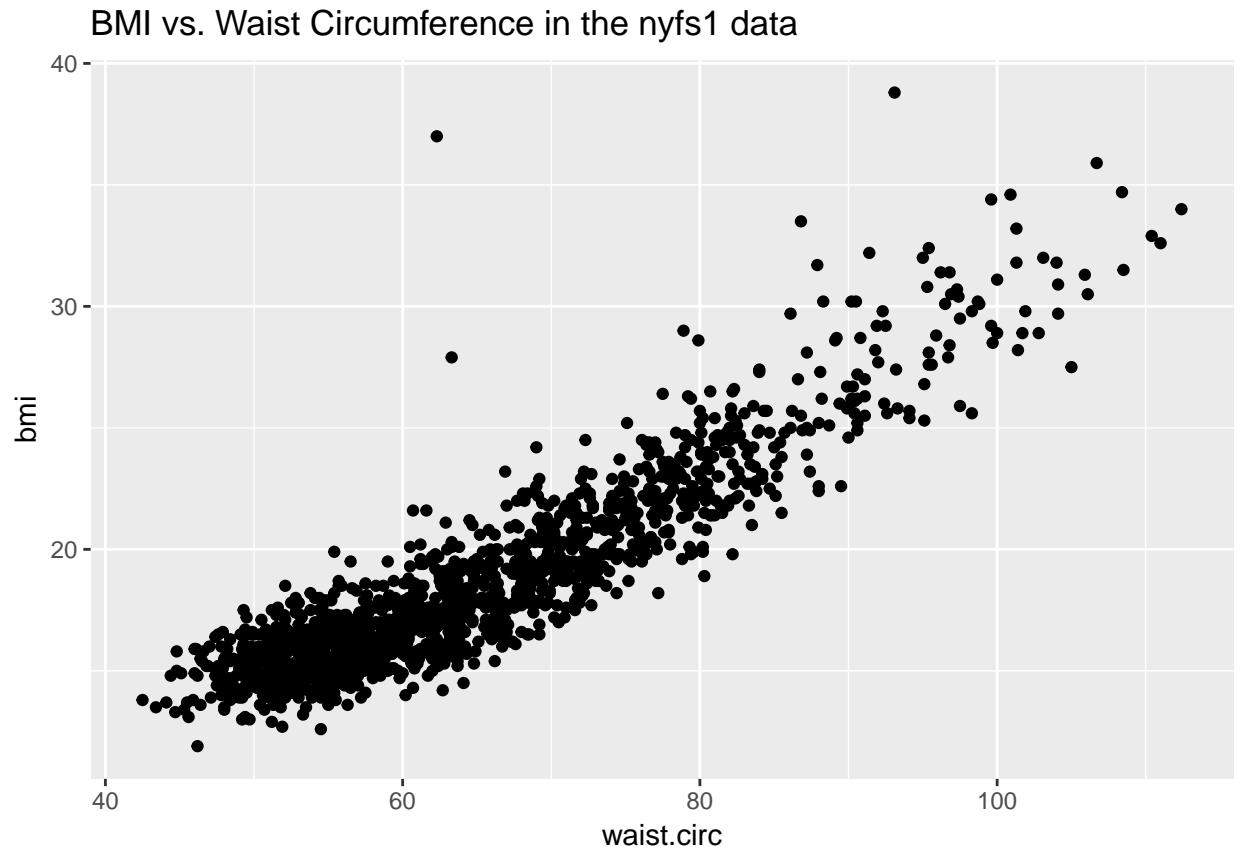
# Chapter 11

## Straight Line Models and Correlation

### 11.1 Assessing A Scatterplot

Let's consider the relationship of `bmi` and `waist.circ` in the `nyfs1` data. We'll begin our investigation, as we always should, by drawing a relevant picture. For the association of two quantitative variables, a **scatterplot** is usually the right start. Each subject in the `nyfs1` data is represented by one of the points below.

```
ggplot(data = nyfs1, aes(x = waist.circ, y = bmi)) +  
  geom_point() +  
  labs(title = "BMI vs. Waist Circumference in the nyfs1 data")
```



Here, I've arbitrarily decided to place `bmi` on the vertical axis, and `waist.circ` on the horizontal. Fitting a prediction model to this scatterplot will then require that we predict `bmi` on the basis of `waist.circ`.

In this case, the pattern appears to be:

1. **direct**, or positive, in that the values of the  $x$  variable (`waist.circ`) increase, so do the values of the  $y$  variable (`bmi`). Essentially, it appears that subjects with larger waist circumferences also have larger BMIs, but we don't know cause and effect here.
2. fairly **linear** in that most of the points cluster around what appears to be a pattern which is well-fitted by a straight line.
3. **strong** in that the range of values for `bmi` associated with any particular value of `waist.circ` is fairly tight. If we know someone's waist circumference, we can pretty accurately predict their BMI, among the subjects in these data.
4. that we see at least one fairly substantial **outlier** value at the upper left of the plot, which I'll identify in the plot below with a red dot.

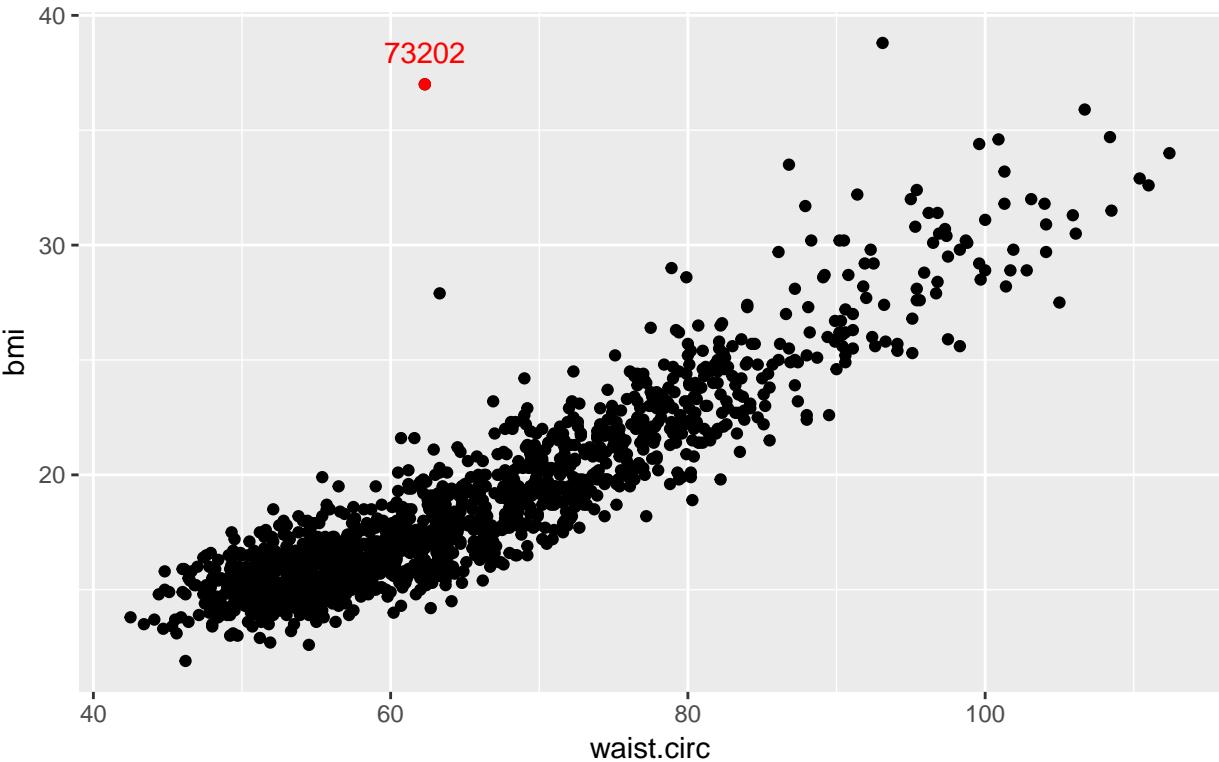
### 11.1.1 Highlighting an unusual point

To highlight the outlier, I'll note that it's the only point with  $\text{BMI} > 35$  and  $\text{waist.circ} < 70$ . So I'll create a subset of the `nyfs1` data containing the point that meets that standard, and then add a red point and a label to the plot.

```
# identify outlier and place it in data frame s1
s1 <- filter(nyfs1, bmi>35 & waist.circ < 70)

ggplot(data = nyfs1, aes(x = waist.circ, y = bmi)) +
  geom_point() +
  # next two lines add outlier color, and then a label
  geom_point(data = s1, col = "red") +
  geom_text(data = s1, label = s1$subject.id, vjust = -1, col = "red") +
  labs(title = "BMI vs. Waist Circumference in the nyfs1 data",
       subtitle = "with outlier labeled by subject ID")
```

### BMI vs. Waist Circumference in the nyfs1 data with outlier labeled by subject ID



```
s1
```

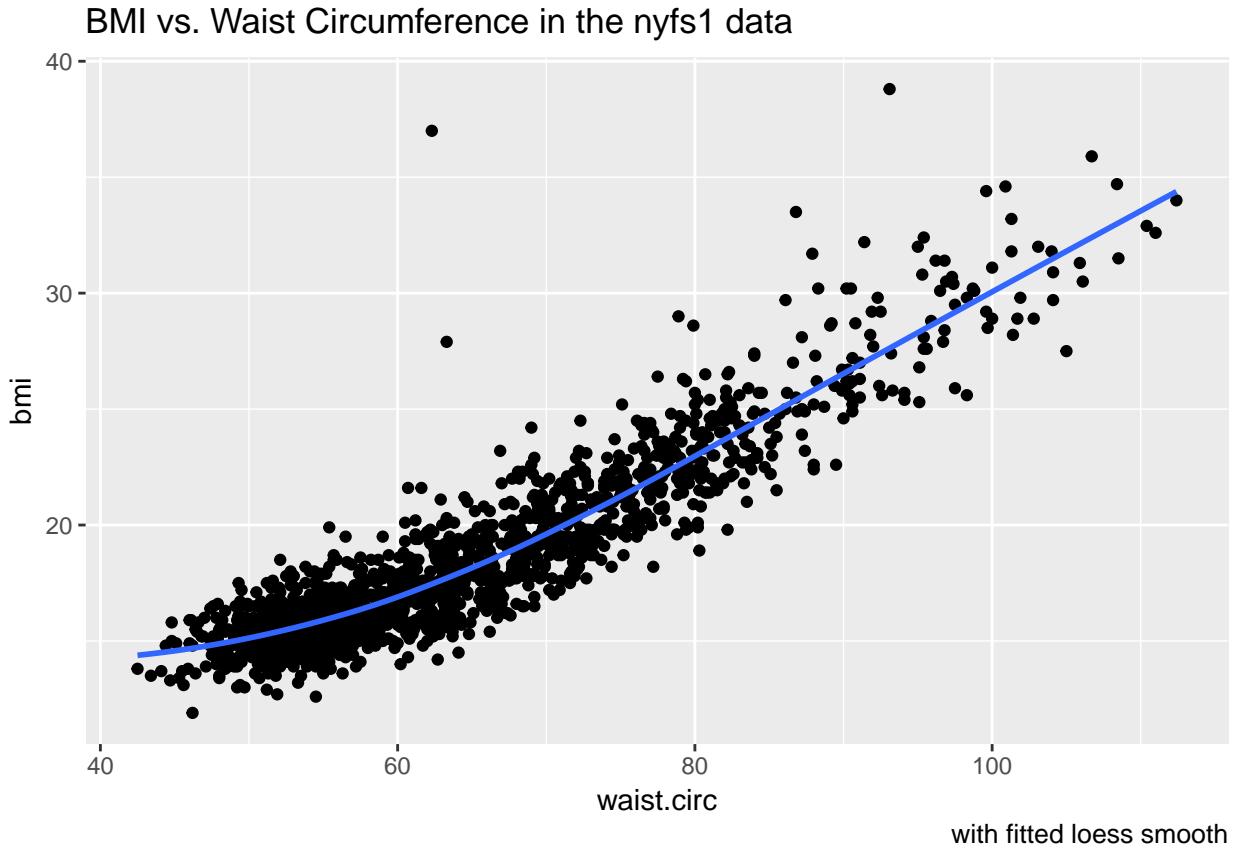
```
# A tibble: 1 x 7
  subject.id   sex age.exam   bmi bmi.cat waist.circ triceps.skinfold
  <int> <fctr>    <int> <dbl> <fctr>      <dbl>             <dbl>
1     73202   Male      13     37   Obese       62.3            7.2
```

Does it seem to you like a straight line model will describe this relationship well?

#### 11.1.2 Adding a Scatterplot Smooth using loess

We'll use the `loess` procedure to fit a smooth curve to the data, which attempts to capture the general pattern.

```
ggplot(data = nyfs1, aes(x = waist.circ, y = bmi)) +
  geom_point() +
  geom_smooth(method = "loess", se = FALSE) +
  labs(title = "BMI vs. Waist Circumference in the nyfs1 data",
       caption = "with fitted loess smooth")
```



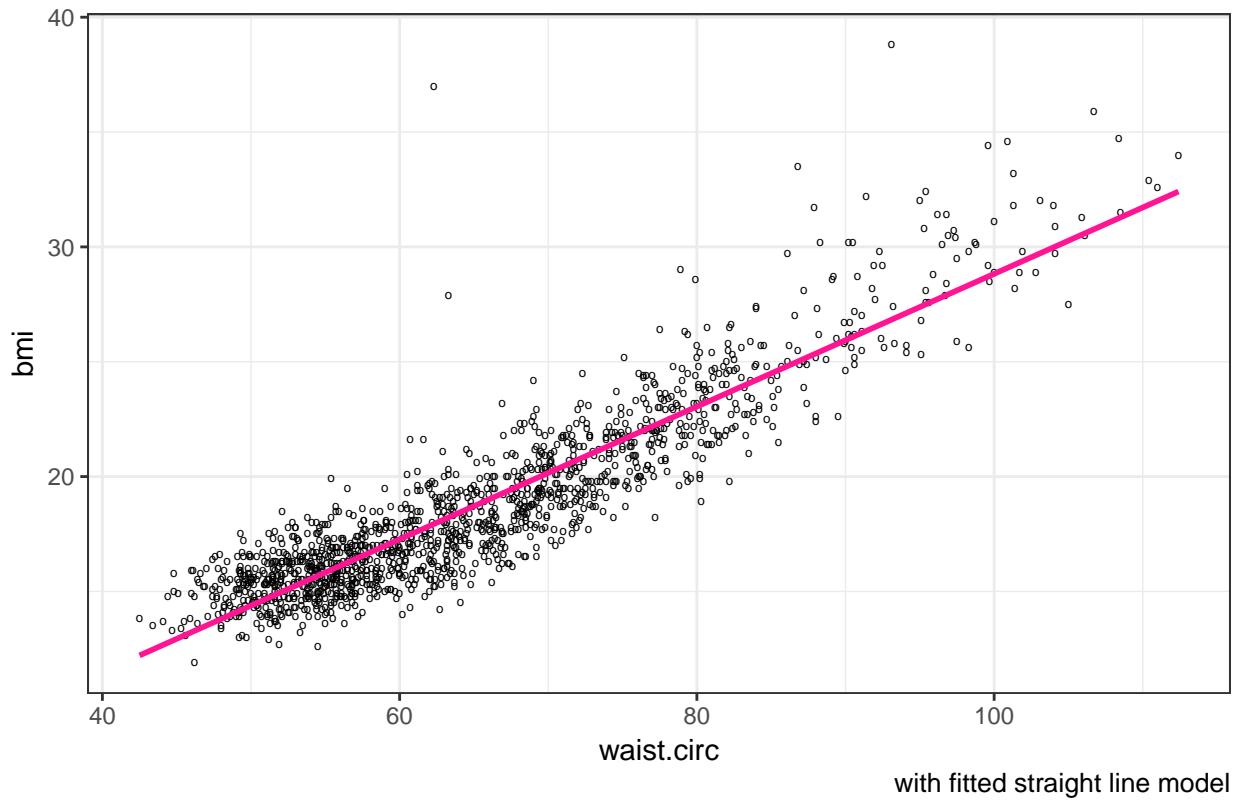
The smooth curve backs up our earlier thought that a straight line might fit the data well. More on the loess smooth in the next chapter.

### 11.1.3 Adding a Straight Line to the Scatterplot

Let's go ahead and add a straight line to the plot, and we'll change the shape of the points to emphasize the fitted line a bit more.

```
ggplot(data = nyfs1, aes(x = waist.circ, y = bmi)) +
  geom_point(shape = "o") +
  geom_smooth(method = "lm", se = FALSE, col = "deeppink") +
  labs(title = "BMI vs. Waist Circumference in the nyfs1 data",
       caption = "with fitted straight line model") +
  theme_bw()
```

### BMI vs. Waist Circumference in the nyfs1 data



How can we, mathematically, characterize that line? As with any straight line, our model equation requires us to specify two parameters: a slope and an intercept (sometimes called the y-intercept.)

#### 11.1.4 What Line Does R Fit?

To identify the equation R used to fit this line (using the method of least squares), we use the `lm` command

```
lm(bmi ~ waist.circ, data = nyfs1)
```

```
Call:  
lm(formula = bmi ~ waist.circ, data = nyfs1)  
  
Coefficients:  
(Intercept)    waist.circ  
-0.0665        0.2889
```

So the fitted line is specified as

$$\text{BMI} = -0.066 + 0.289 \text{ Waist Circumference}$$

A detailed summary of the fitted linear regression model is also available.

```
summary(lm(bmi ~ waist.circ, data = nyfs1))
```

```
Call:
```

```

lm(formula = bmi ~ waist.circ, data = nyfs1)

Residuals:
    Min      1Q Median      3Q     Max 
-4.234 -1.094 -0.074  0.925 19.066 

Coefficients:
            Estimate Std. Error t value Pr(>|t|)    
(Intercept) -0.0665    0.2329   -0.29    0.78    
waist.circ    0.2889    0.0035   82.55 <2e-16 ***  
---
Signif. codes:  0 '***' 0.001 '**' 0.01 '*' 0.05 '.' 0.1 ' ' 1

Residual standard error: 1.69 on 1414 degrees of freedom
Multiple R-squared:  0.828, Adjusted R-squared:  0.828 
F-statistic: 6.81e+03 on 1 and 1414 DF,  p-value: <2e-16

```

We'll spend a lot of time working with these regression summaries, especially in Part C of the course.

For now, it will suffice to understand the following:

- The outcome variable in this model is **bmi**, and the predictor variable is **waist.circ**.
- The straight line model for these data fitted by least squares is  $\text{bmi} = -0.066 + 0.289 \text{waist.circ}$
- The slope of **waist.circ** is positive, which indicates that as **waist.circ** increases, we expect that **bmi** will also increase. Specifically, we expect that for every additional cm of waist circumference, the BMI will be 0.289 kg/m<sup>2</sup> larger.
- The multiple R-squared (squared correlation coefficient) is 0.828, which implies that 82.8% of the variation in **bmi** is explained using this linear model with **waist.circ**. It also implies that the Pearson correlation between force and height is the square root of 0.828, or 0.91. More on the Pearson correlation soon.

So, if we plan to use a simple (least squares) linear regression model to describe BMI as a function of waist circumference, does it look like a least squares model is likely to be an effective choice here?

## 11.2 Correlation Coefficients

Two different correlation measures are worth our immediate attention.

- The one most often used is called the *Pearson* correlation coefficient, and is symbolized with the letter *r* or sometimes the Greek letter rho ( $\rho$ ).
- Another tool is the Spearman rank correlation coefficient, also occasionally symbolized by  $\rho$ .

For the **nyfs1** data, the Pearson correlation of **bmi** and **waist.circ** can be found using the **cor()** function.

```
cor(nyfs1$bmi, nyfs1$waist.circ)
```

```
[1] 0.91
```

```
nyfs1 %>%
  select(bmi, waist.circ) %>%
  cor()
```

	bmi	waist.circ
bmi	1.00	0.91
waist.circ	0.91	1.00

Note that the correlation of any variable with itself is 1, and that the correlation of `bmi` with `waist.circ` is the same regardless of whether you enter `bmi` first or `waist.circ` first.

## 11.3 The Pearson Correlation Coefficient

Suppose we have  $n$  observations on two variables, called  $X$  and  $Y$ . The Pearson correlation coefficient assesses how well the relationship between  $X$  and  $Y$  can be described using a linear function.

- The Pearson correlation is **dimension-free**.
- It falls between -1 and +1, with the extremes corresponding to situations where all the points in a scatterplot fall exactly on a straight line with negative and positive slopes, respectively.
- A Pearson correlation of zero corresponds to the situation where there is no linear association.
- Unlike the estimated slope in a regression line, the sample correlation coefficient is symmetric in  $X$  and  $Y$ , so it does not depend on labeling one of them ( $Y$ ) the response variable, and one of them ( $X$ ) the predictor.

Suppose we have  $n$  observations on two variables, called  $X$  and  $Y$ , where  $\bar{X}$  is the sample mean of  $X$  and  $s_x$  is the standard deviation of  $X$ . The **Pearson** correlation coefficient  $r_{XY}$  is:

$$r_{XY} = \frac{1}{n-1} \sum_{i=1}^n \left( \frac{x_i - \bar{x}}{s_x} \right) \left( \frac{y_i - \bar{y}}{s_y} \right)$$

## 11.4 A simulated example

The `correx1` data file contains six different sets of (x,y) points, identified by the `set` variable.

```
correx1 <- read.csv("data/correx1.csv") %>%tbl_df
summary(correx1)
```

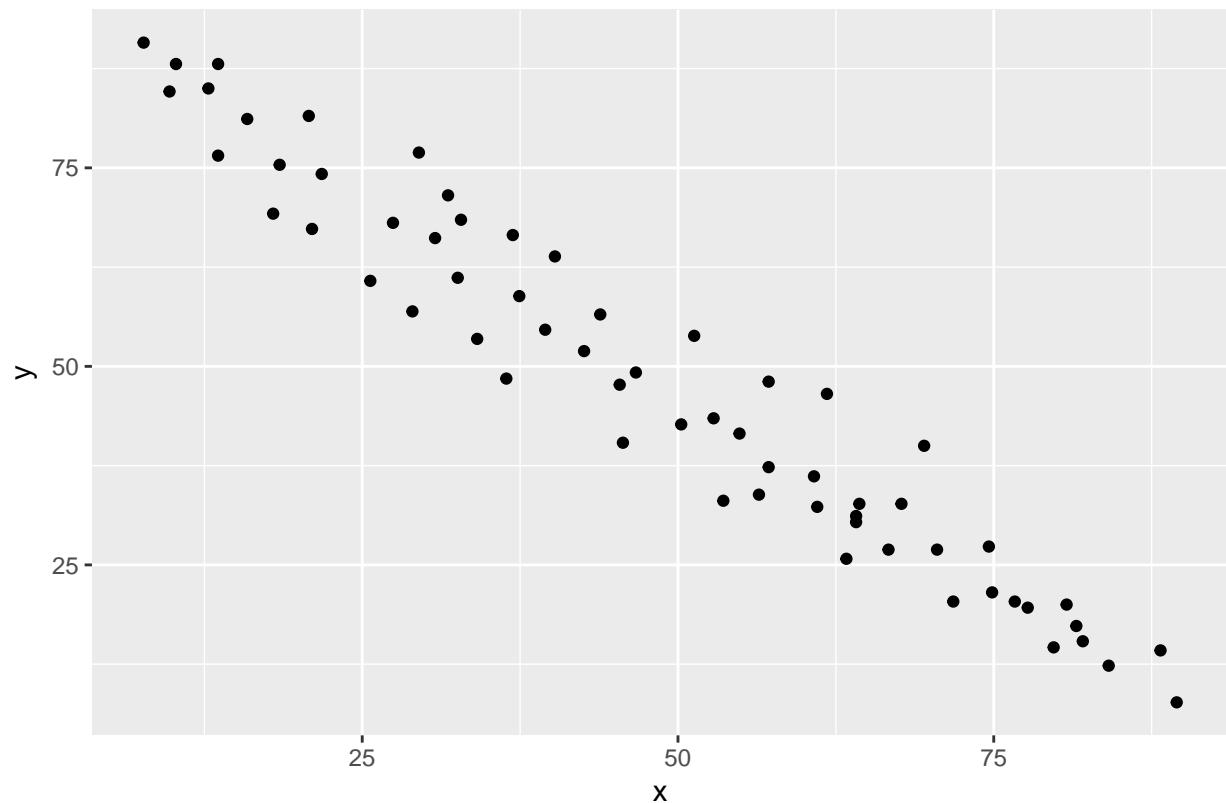
	set	x	y
Alex	:62	Min. : 5.9	Min. : 7.3
Bonnie	:37	1st Qu.:29.5	1st Qu.:30.4
Colin	:36	Median :46.2	Median :46.9
Danielle	:70	Mean :46.5	Mean :49.1
Earl	:15	3rd Qu.:63.3	3rd Qu.:68.1
Fiona	:57	Max. :98.2	Max. :95.4

### 11.4.1 Data Set Alex

Let's start by working with the **Alex** data set.

```
ggplot(filter(correx1, set == "Alex"), aes(x = x, y = y)) +
  geom_point() +
  labs(title = "correx1: Data Set Alex")
```

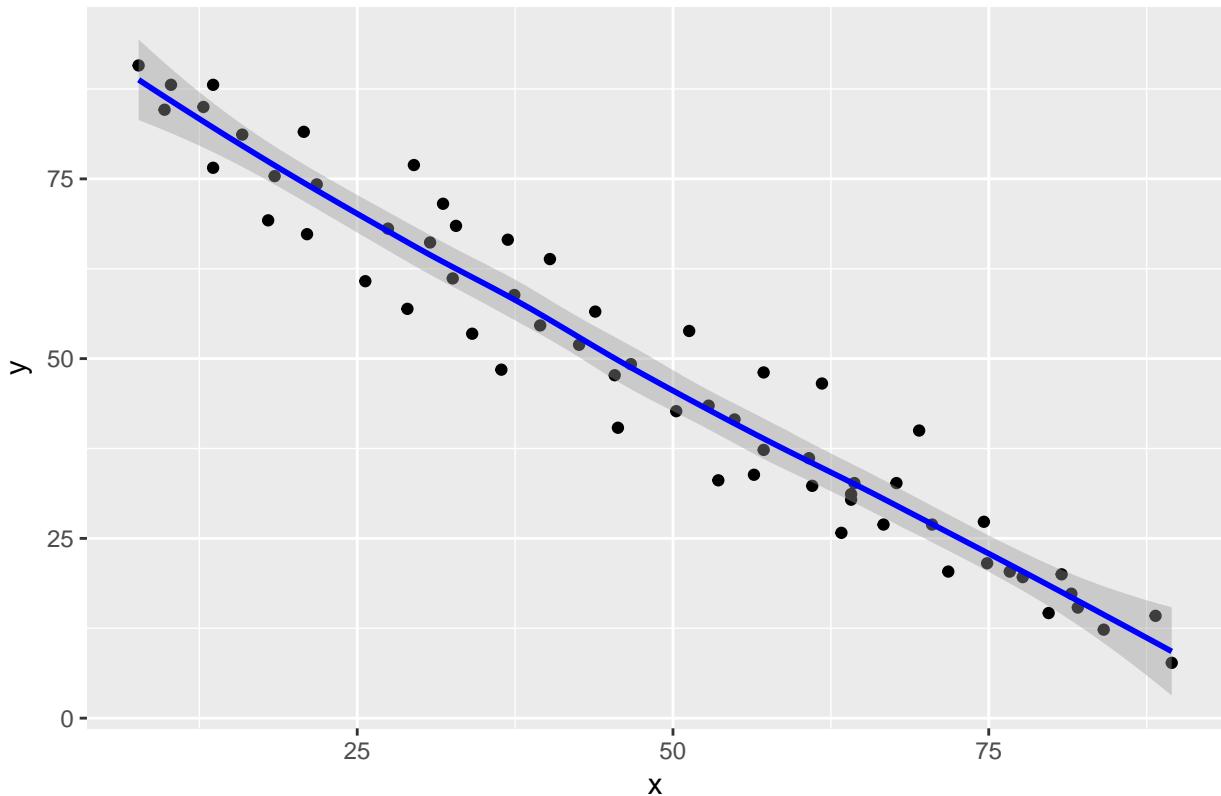
## correx1: Data Set Alex



```
ggplot(filter(correx1, set == "Alex"), aes(x = x, y = y)) +  
  geom_point() +  
  geom_smooth(col = "blue") +  
  labs(title = "correx1: Alex, with loess smooth")
```

```
`geom_smooth()` using method = 'loess'
```

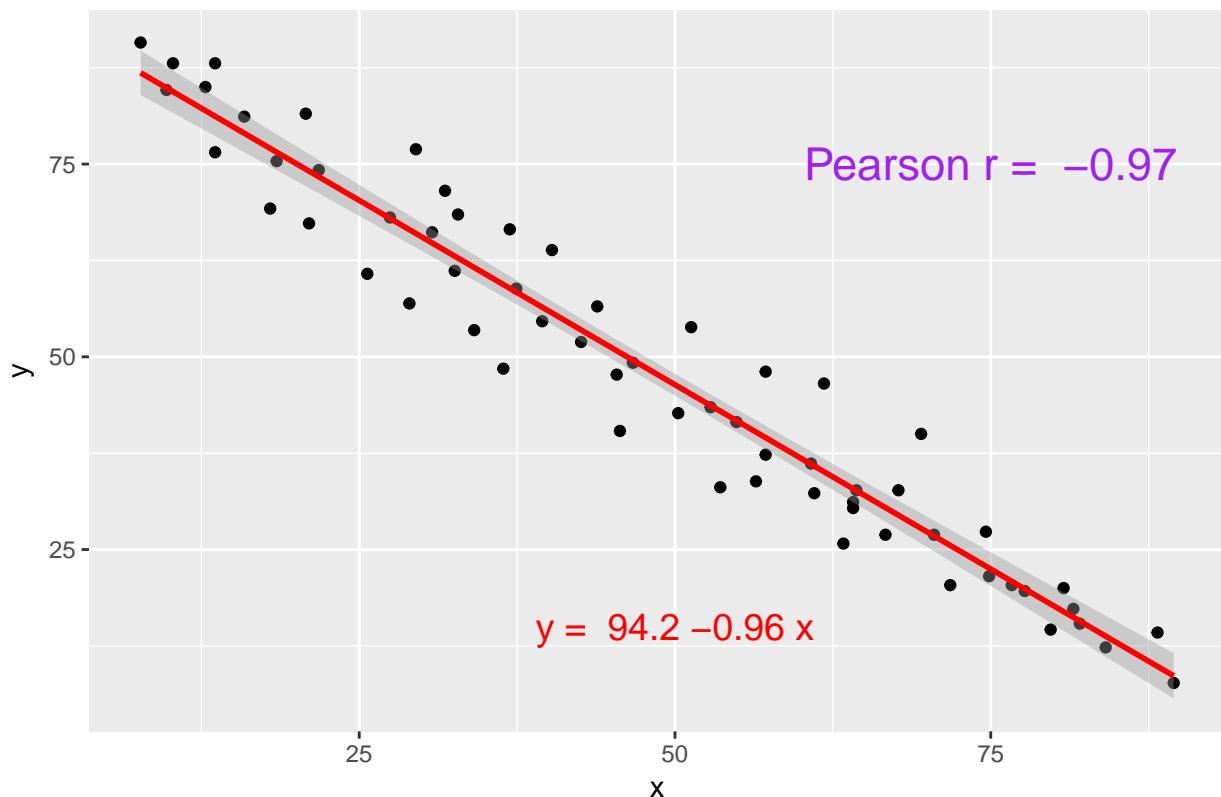
## correx1: Alex, with loess smooth



```
setA <- filter(correx1, set == "Alex")

ggplot(setA, aes(x = x, y = y)) +
  geom_point() +
  geom_smooth(method = "lm", col = "red") +
  labs(title = "correx1: Alex, with Fitted Linear Model") +
  annotate("text", x = 75, y = 75, col = "purple", size = 6,
          label = paste("Pearson r = ", signif(cor(setA$x, setA$y),3))) +
  annotate("text", x = 50, y = 15, col = "red", size = 5,
          label = paste("y = ", signif(coef(lm(setA$y ~ setA$x))[1],3),
                        signif(coef(lm(setA$y ~ setA$x))[2],2), "x"))
```

correx1: Alex, with Fitted Linear Model

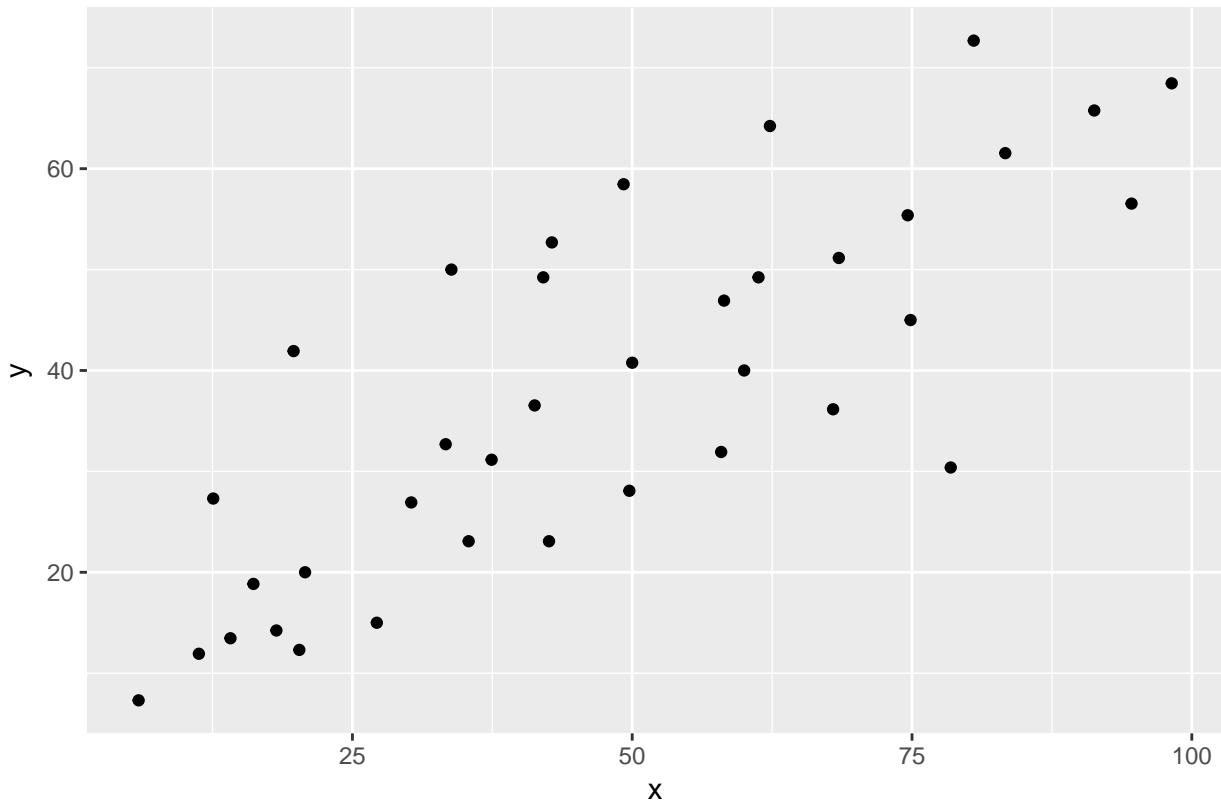


#### 11.4.2 Data Set Bonnie

```
setB <- dplyr::filter(correx1, set == "Bonnie")

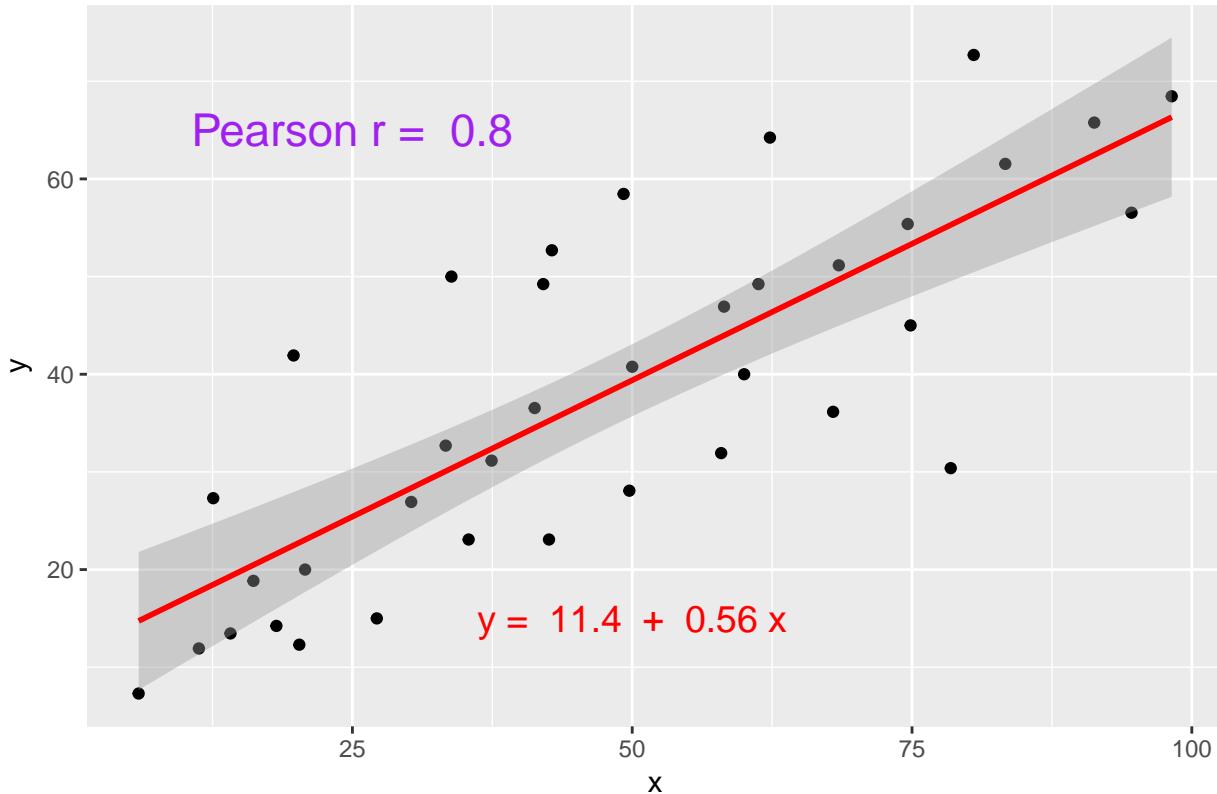
ggplot(setB, aes(x = x, y = y)) +
  geom_point() +
  labs(title = "correx1: Data Set Bonnie")
```

## correx1: Data Set Bonnie



```
ggplot(setB, aes(x = x, y = y)) +
  geom_point() +
  geom_smooth(method = "lm", col = "red") +
  labs(title = "correx1: Bonnie, with Fitted Linear Model") +
  annotate("text", x = 25, y = 65, col = "purple", size = 6,
          label = paste("Pearson r = ", signif(cor(setB$x, setB$y), 2))) +
  annotate("text", x = 50, y = 15, col = "red", size = 5,
          label = paste("y = ", signif(coef(lm(setB$y ~ setB$x))[1], 3),
                        " + ",
                        signif(coef(lm(setB$y ~ setB$x))[2], 2), "x"))
```

### correx1: Bonnie, with Fitted Linear Model



#### 11.4.3 Correlations for All Six Data Sets in the Correx1 Example

Let's look at the Pearson correlations associated with each of the six data sets contained in the `correx1` example.

```
tab1 <- correx1 %>%
  group_by(set) %>%
  summarise("Pearson r" = round(cor(x, y, use="complete"), 2))

knitr::kable(tab1)
```

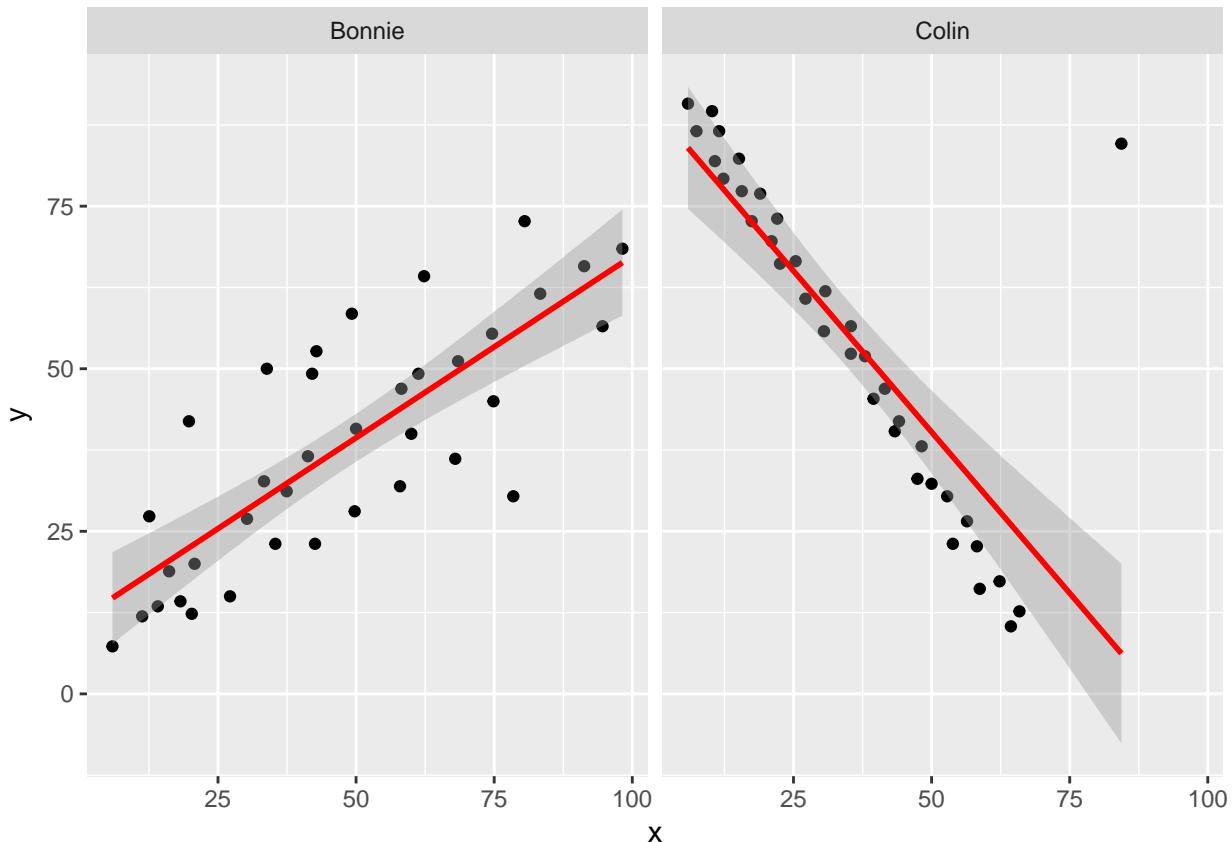
set	Pearson r
Alex	-0.97
Bonnie	0.80
Colin	-0.80
Danielle	0.00
Earl	-0.01
Fiona	0.00

#### 11.4.4 Data Set Colin

It looks like the picture for Colin should be very similar (in terms of scatter) to the picture for Bonnie, except that Colin will have a negative slope, rather than the positive one Bonnie has. Is that how this plays out?

```
setBC <- filter(correx1, set == "Bonnie" | set == "Colin")

ggplot(setBC, aes(x = x, y = y)) +
  geom_point() +
  geom_smooth(method = "lm", col = "red") +
  facet_wrap(~ set)
```

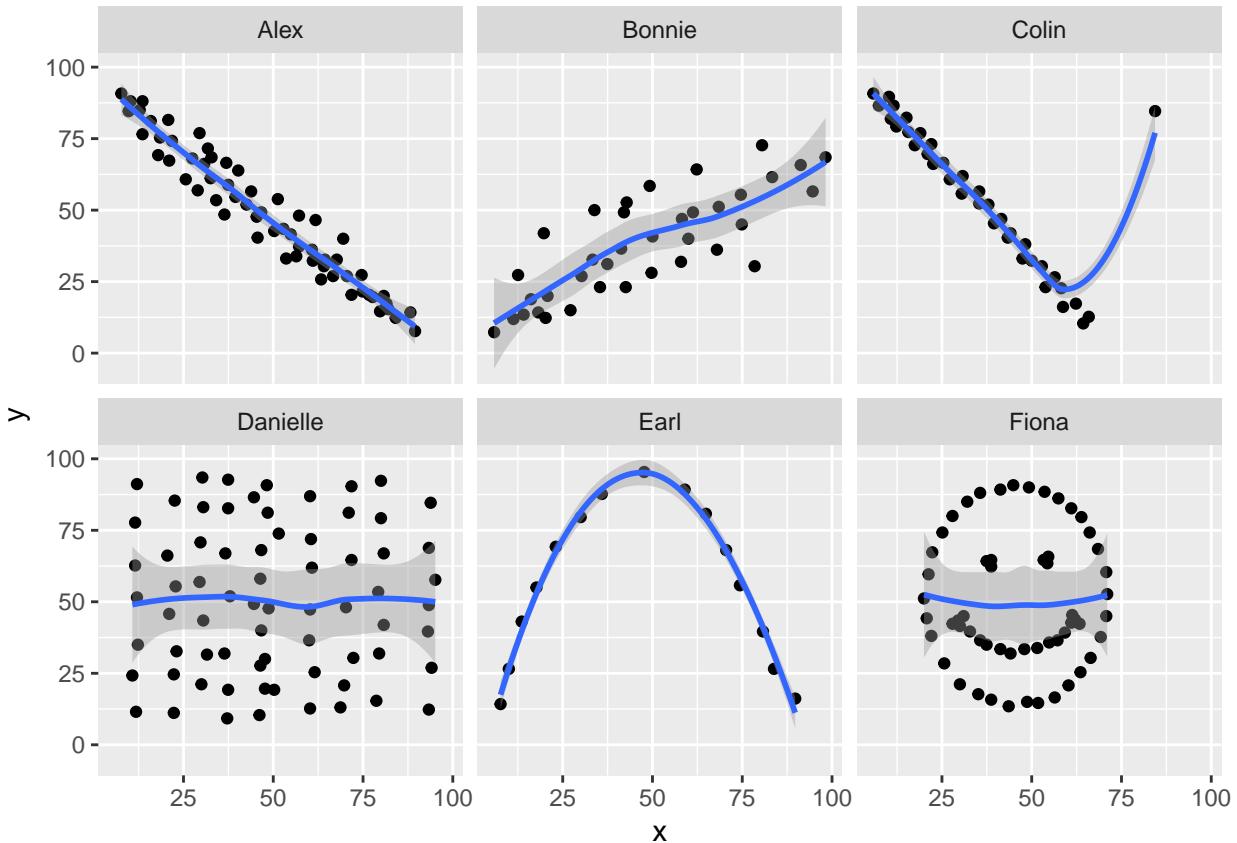


Uh, oh. It looks like the point in Colin at the top right is twisting what would otherwise be a very straight regression model with an extremely strong negative correlation. There's no better way to look for outliers than to examine the scatterplot.

#### 11.4.5 Draw the Picture!

We've seen that Danielle, Earl and Fiona all show Pearson correlations of essentially zero. However, the three data sets look very different in a scatterplot.

```
ggplot(correx1, aes(x = x, y = y)) +
  geom_point() +
  geom_smooth(method = "loess") +
  facet_wrap(~ set)
```



When we learn that the correlation is zero, we tend to assume we have a picture like the Danielle data set. If Danielle were our real data, we might well think that  $x$  would be of little use in predicting  $y$ .

- But what if our data looked like Earl? In the Earl data set,  $x$  is incredibly helpful in predicting  $y$ , but we can't use a straight line model - instead, we need a non-linear modeling approach.
- You'll recall that the Fiona data set also had a Pearson correlation of zero. But here, the picture is rather more interesting.

So, remember, draw the d%\$# picture whenever you make use of a summary statistic, like a correlation coefficient, or linear model.

```
rm(setA, setB, setBC, tab1)
```

## 11.5 Estimating Correlation from Scatterplots

The correx2 data set is designed to help you calibrate yourself a bit in terms of estimating a correlation from a scatterplot. There are 11 data sets buried within the correx2 example, and they are labeled by their Pearson correlation coefficients, ranging from  $r = 0.01$  to  $r = 0.999$

```
correx2 <- read.csv("data/correx2.csv") %>%tbl_df

correx2 %>%
  group_by(set) %>%
  summarise(cor = round(cor(x, y, use="complete"),3))
```

```
# A tibble: 11 x 2
  set     cor
```

```

<fctr> <dbl>
1 Set 01 0.010
2 Set 10 0.102
3 Set 20 0.202
4 Set 30 0.301
5 Set 40 0.403
6 Set 50 0.499
7 Set 60 0.603
8 Set 70 0.702
9 Set 80 0.799
10 Set 90 0.902
11 Set 999 0.999

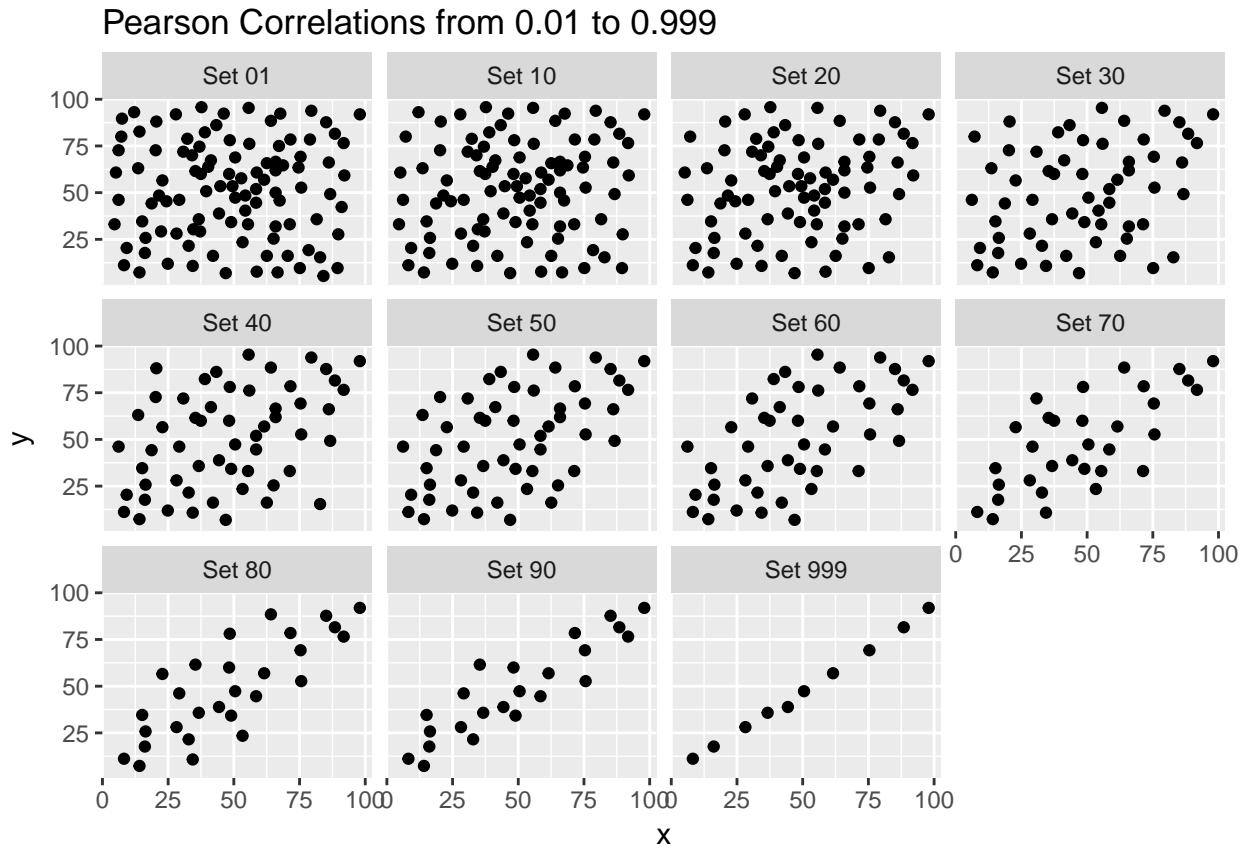
```

Here is a plot of the 11 data sets, showing the increase in correlation from 0.01 (in Set 01) to 0.999 (in Set 999).

```

ggplot(correx2, aes(x = x, y = y)) +
  geom_point() +
  facet_wrap(~ set) +
  labs(title = "Pearson Correlations from 0.01 to 0.999")

```



Note that R will allow you to fit a straight line model to any of these relationships, no matter how appropriate it might be to do so.

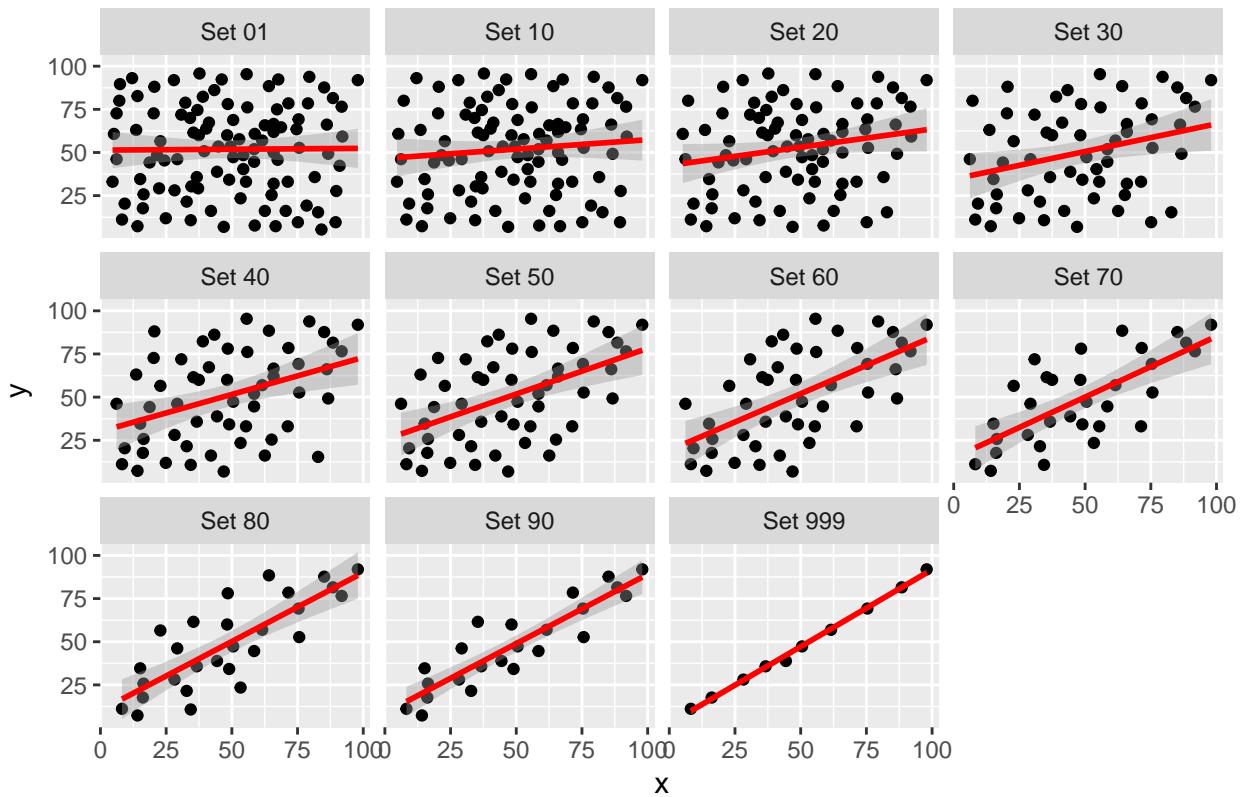
```

ggplot(correx2, aes(x = x, y = y)) +
  geom_point() +
  geom_smooth(method = "lm", col = "red") +
  facet_wrap(~ set) +

```

```
labs(title = "R will fit a straight line to anything.")
```

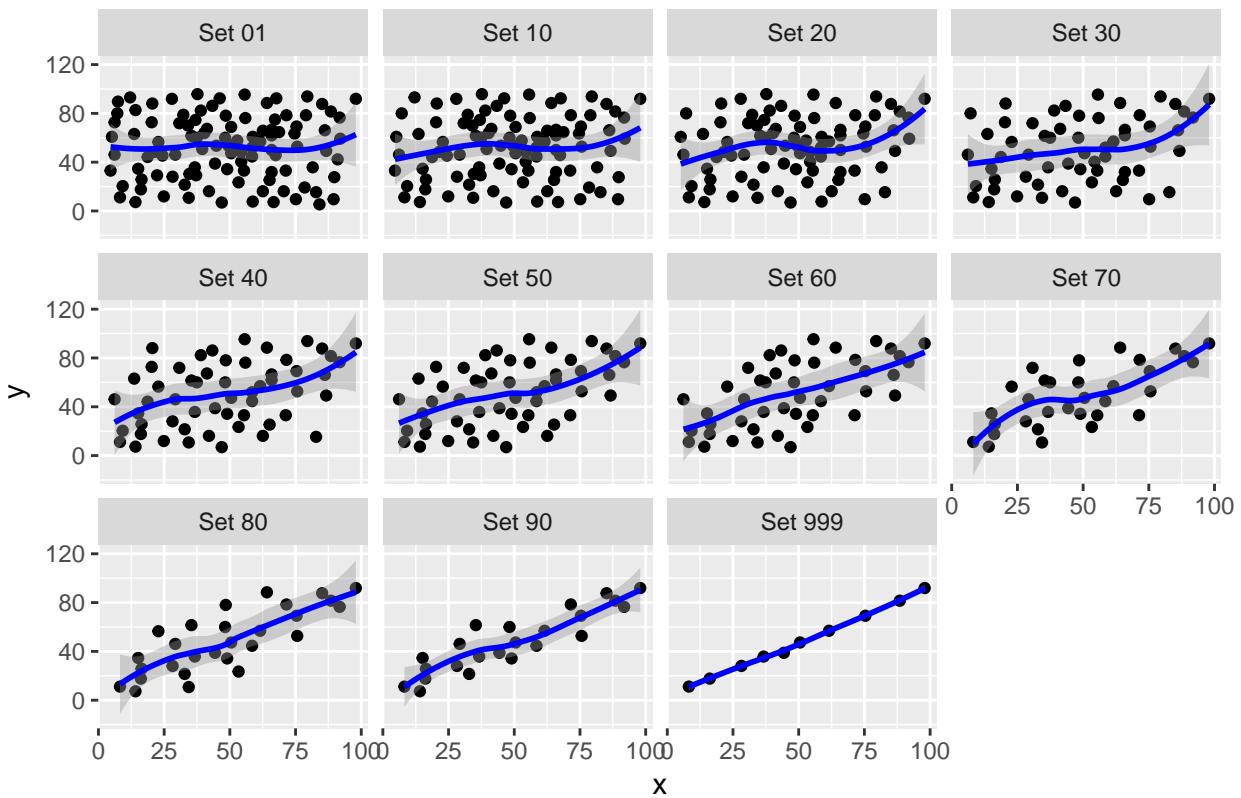
R will fit a straight line to anything.



```
ggplot(correx2, aes(x = x, y = y)) +
  geom_point() +
  geom_smooth(col = "blue") +
  facet_wrap(~ set) +
  labs(title = "Even if a loess smooth suggests non-linearity.")
```

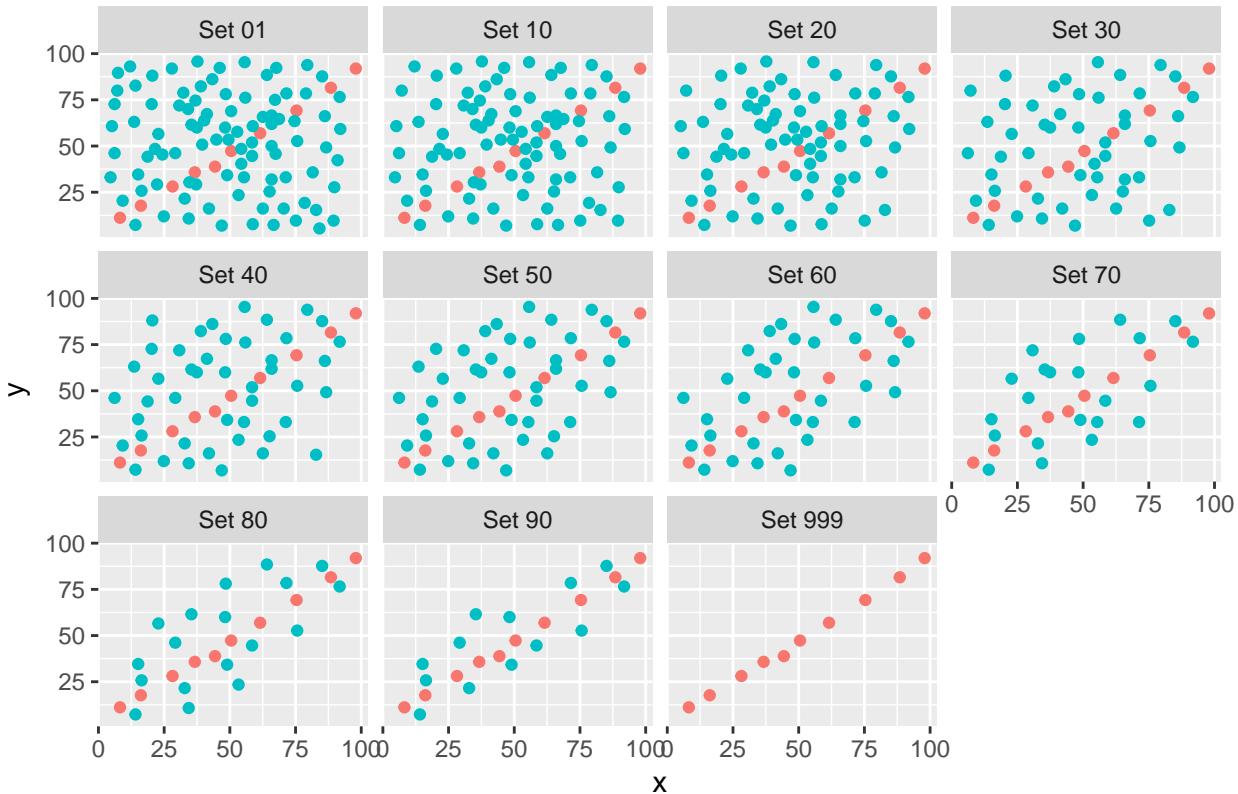
`geom\_smooth()` using method = 'loess'

Even if a loess smooth suggests non-linearity.



```
ggplot(correx2, aes(x = x, y = y, color = factor(group))) +
  geom_point() +
  guides(color = "none") +
  facet_wrap(~ set) +
  labs(title = "Note: The same 10 points (in red) are in each plot.")
```

Note: The same 10 points (in red) are in each plot.



Note that the same 10 points are used in each of the data sets. It's always possible that a lurking subgroup of the data within a scatterplot follows a very strong linear relationship. This is why it's so important (and difficult) not to go searching for such a thing without a strong foundation of logic, theory and prior empirical evidence.

## 11.6 The Spearman Rank Correlation

The Spearman rank correlation coefficient is a rank-based measure of statistical dependence that assesses how well the relationship between X and Y can be described using a **monotone function** even if that relationship is not linear.

- A monotone function preserves order, that is, Y must either be strictly increasing as X increases, or strictly decreasing as X increases.
- A Spearman correlation of 1.0 indicates simply that as X increases, Y always increases.
- Like the Pearson correlation, the Spearman correlation is dimension-free, and falls between -1 and +1.
- A positive Spearman correlation corresponds to an increasing (but not necessarily linear) association between X and Y, while a negative Spearman correlation corresponds to a decreasing (but again not necessarily linear) association.

### 11.6.1 Spearman Formula

To calculate the Spearman rank correlation, we take the ranks of the X and Y data, and then apply the usual Pearson correlation. To find the ranks, sort X and Y into ascending order, and then number them from 1 (smallest) to n (largest). In the event of a tie, assign the average rank to the tied subjects.

### 11.6.2 Comparing Pearson and Spearman Correlations

Let's look at the `nyfs1` data again.

```
cor(nyfs1$bmi, nyfs1$waist.circ)

[1] 0.91

cor(nyfs1$bmi, nyfs1$waist.circ, method = "spearman")
```

```
[1] 0.889

nyfs1 %>%
  select(bmi, waist.circ) %>%
  cor(., method = "spearman")
```

	bmi	waist.circ
bmi	1.000	0.889
waist.circ	0.889	1.000

The Spearman and Pearson correlations are not especially different in this case.

### 11.6.3 Spearman vs. Pearson Example 1

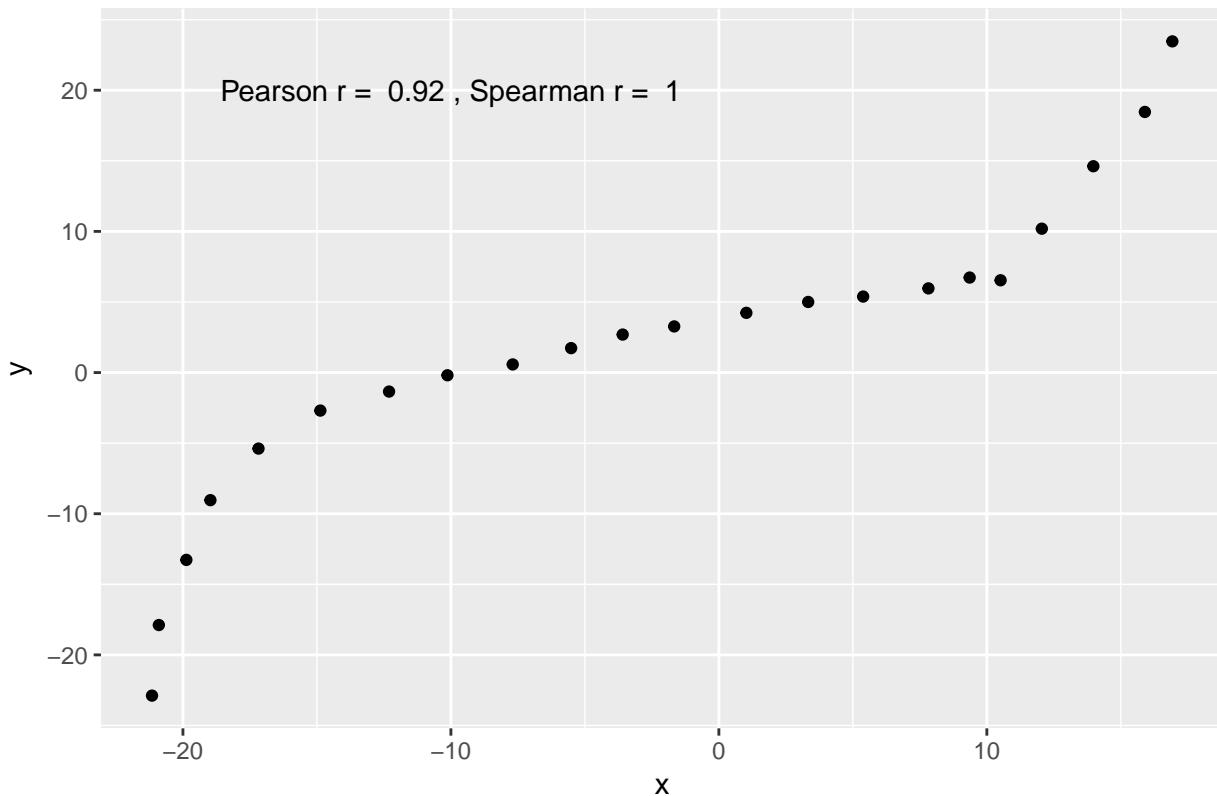
The next few plots describe relationships where we anticipate the Pearson and Spearman correlations might differ in their conclusions.

```
spear1 <- read.csv("data/spear1.csv")
spear2 <- read.csv("data/spear2.csv")
spear3 <- read.csv("data/spear3.csv")
spear4 <- read.csv("data/spear4.csv")
# used read.csv above because these are just toy examples with
# two columns per data set and no row numbering
```

Example 1 shows a function where the Pearson correlation is 0.925 (a strong but not perfect linear relation), but the Spearman correlation is `signif(cor(spear1$x, spear1$y, method = "spearman"), 2)` because the relationship is monotone, even though it is not perfectly linear.

```
ggplot(spear1, aes(x = x, y = y)) +
  geom_point() +
  labs(title = "Spearman vs. Pearson, Example 1") +
  annotate("text", x = -10, y = 20,
    label = paste("Pearson r = ",
      signif(cor(spear1$x, spear1$y), 2),
      ", Spearman r = ",
      signif(cor(spear1$x, spear1$y, method = "spearman"), 2)))
```

### Spearman vs. Pearson, Example 1



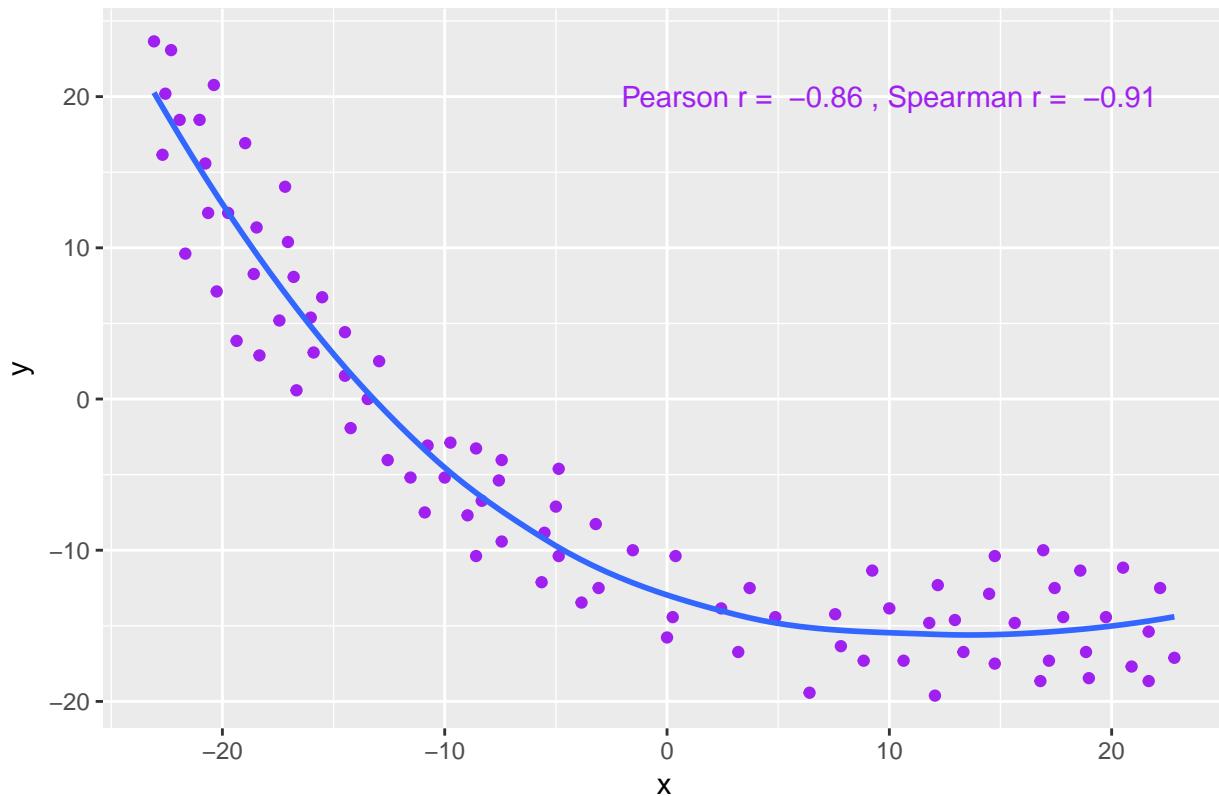
So, a positive Spearman correlation corresponds to an increasing (but not necessarily linear) association between x and y.

#### 11.6.4 Spearman vs. Pearson Example 2

Example 2 shows that a negative Spearman correlation corresponds to a decreasing (but, again, not necessarily linear) association between x and y.

```
ggplot(spear2, aes(x = x, y = y)) +
  geom_point(col = "purple") +
  geom_smooth(method = "loess", se = FALSE) +
  labs(title = "Spearman vs. Pearson, Example 2") +
  annotate("text", x = 10, y = 20, col = "purple",
    label = paste("Pearson r = ",
      signif(cor(spear2$x, spear2$y),2),
      ", Spearman r = ",
      signif(cor(spear2$x, spear2$y, method = "spearman"),2)))
```

### Spearman vs. Pearson, Example 2



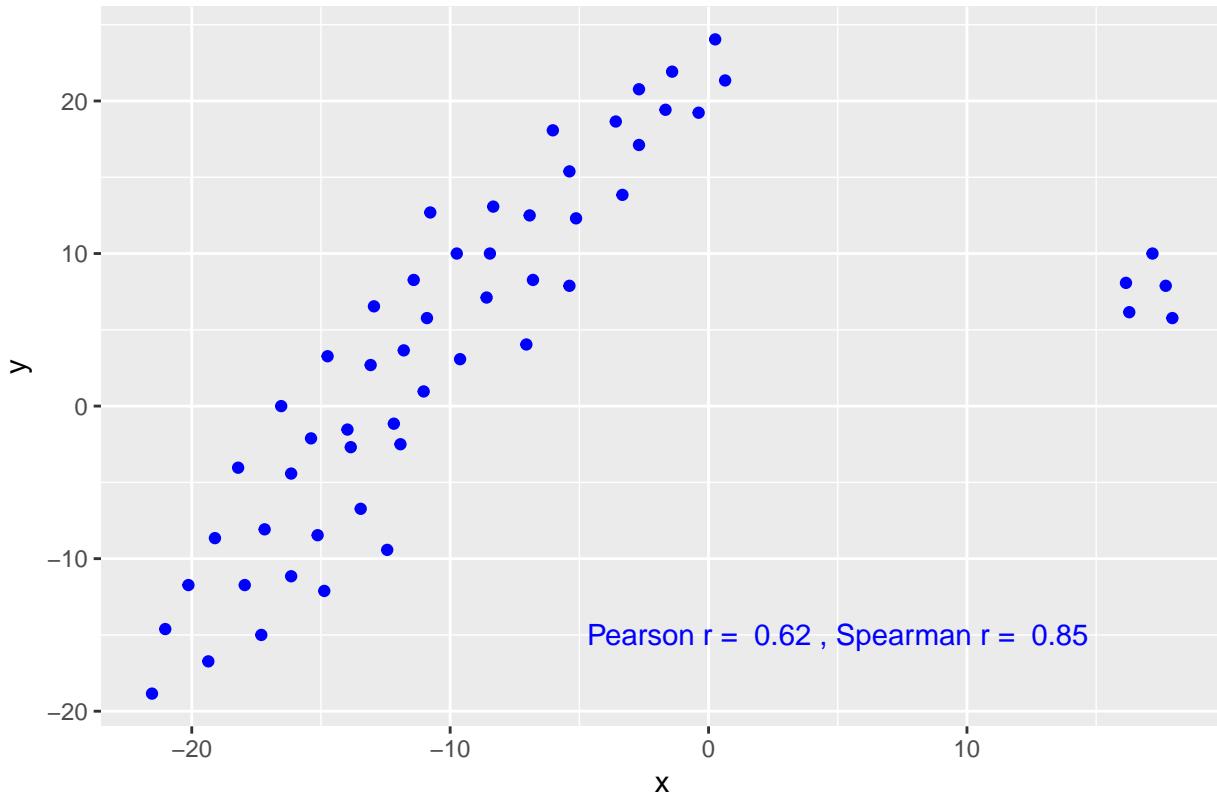
### 11.6.5 Spearman vs. Pearson Example 3

The Spearman correlation is less sensitive than the Pearson correlation to strong outliers that are unusual on either the X or Y axis, or both. That is because the Spearman rank coefficient limits the outlier to the value of its rank.

In Example 3, for instance, the Spearman correlation reacts much less to the outliers around  $X = 12$  than does the Pearson correlation.

```
ggplot(spear3, aes(x = x, y = y)) +
  geom_point(col = "blue") +
  labs(title = "Spearman vs. Pearson, Example 3") +
  annotate("text", x = 5, y = -15, col = "blue",
           label = paste("Pearson r = ",
                         signif(cor(spear3$x, spear3$y),2),
                         ", Spearman r = ",
                         signif(cor(spear3$x, spear3$y, method = "spearman"),2)))
```

### Spearman vs. Pearson, Example 3

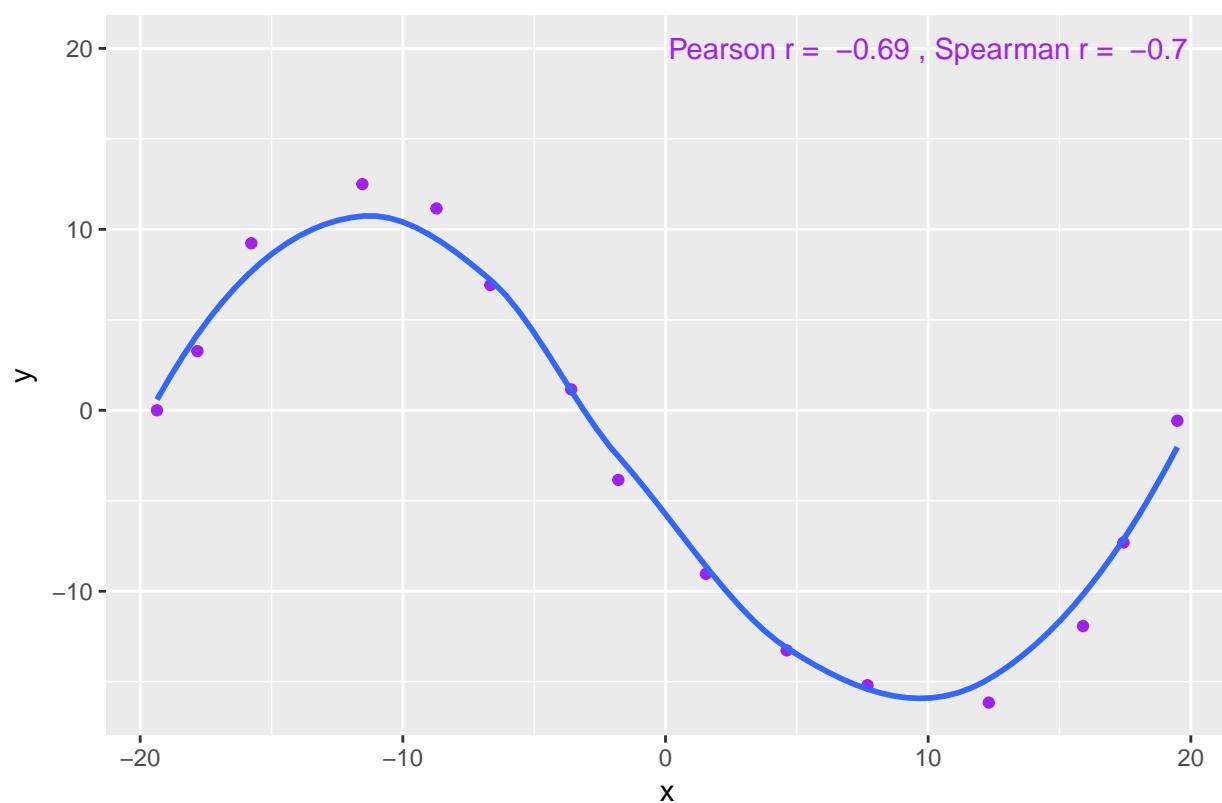


### 11.6.6 Spearman vs. Pearson Example 4

The use of a Spearman correlation is no substitute for looking at the data. For non-monotone data like what we see in Example 4, neither the Spearman nor the Pearson correlation alone provides much guidance, and just because they are (essentially) telling you the same thing, that doesn't mean what they're telling you is all that helpful.

```
ggplot(spear4, aes(x = x, y = y)) +
  geom_point(col = "purple") +
  geom_smooth(method = "loess", se = FALSE) +
  labs(title = "Spearman vs. Pearson, Example 4") +
  annotate("text", x = 10, y = 20, col = "purple",
    label = paste("Pearson r = ",
      signif(cor(spear4$x, spear4$y), 2),
      ", Spearman r = ",
      signif(cor(spear4$x, spear4$y, method = "spearman"), 2)))
```

## Spearman vs. Pearson, Example 4





# Chapter 12

## Studying Crab Claws (crabs)

For our next example, we'll consider a study from zoology, specifically carcinology - the study of crustaceans. My source for these data is Chapter 7 in Ramsey and Schafer (2002) which drew the data from a figure in Yamada and Boulding (1998).

The available data are the mean closing forces (in Newtons) and the propodus heights (mm) of the claws on 38 crabs that came from three different species. The *propodus* is the segment of the crab's clawed leg with an immovable finger and palm.

This was part of a study of the effects that predatory intertidal crab species have on populations of snails. The three crab species under study are:

- 14 *Hemigrapsus nudus*, also called the purple shore crab (14 crabs)
- 12 *Lophopanopeus bellus*, also called the black-clawed pebble crab, and
- 12 *Cancer productus*, one of several species of red rock crabs (12)

```
crabs <- read.csv("data/crabs.csv") %>%tbl_df  
  
crabs  
  
# A tibble: 38 x 4  
  crab           species force height  
  <int>        <fctr>  <dbl>  <dbl>  
1   1  Hemigrapsus nudus    4.0    8.0  
2   2 Lophopanopeus bellus  15.1    7.9  
3   3  Cancer productus    5.0    6.7  
4   4 Lophopanopeus bellus   2.9    6.6  
5   5  Hemigrapsus nudus    3.2    5.0  
6   6  Hemigrapsus nudus    9.5    7.9  
7   7  Cancer productus   22.5    9.4  
8   8  Hemigrapsus nudus    7.4    8.3  
9   9  Cancer productus   14.6   11.2  
10  10 Lophopanopeus bellus   8.7    8.6  
# ... with 28 more rows
```

Here's a quick summary of the data. Take care to note the useless results for the first two variables. At least the function flags with a \* those variables it thinks are non-numeric.

```
psych::describe(crabs)  
  
vars n  mean     sd median trimmed   mad min  max range skew  
crab    1 38 19.50 11.11  19.50   19.50 14.08   1 38.0 37.0 0.00
```

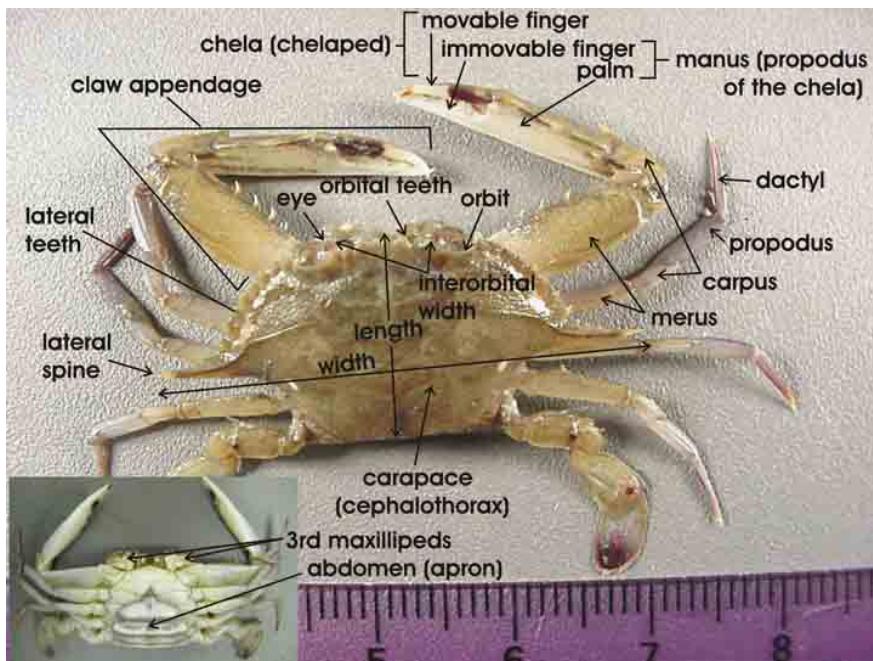


Figure 12.1: Source: <http://txmarspecies.tamug.edu/crustglossary.cfm>

species*	2	38	2.00	0.81	2.00	2.00	1.48	1	3.0	2.0	0.00
force	3	38	12.13	8.98	8.70	11.53	9.04	2	29.4	27.4	0.47
height	4	38	8.81	2.23	8.25	8.78	2.52	5	13.1	8.1	0.19
kurtosis			se								
crab			-1.30	1.80							
species*			-1.50	0.13							
force			-1.25	1.46							
height			-1.14	0.36							

Actually, we're more interested in these results after grouping by species.

```
crabs %>%
  group_by(species) %>%
  summarise(n = n(), median(force), median(height))
```

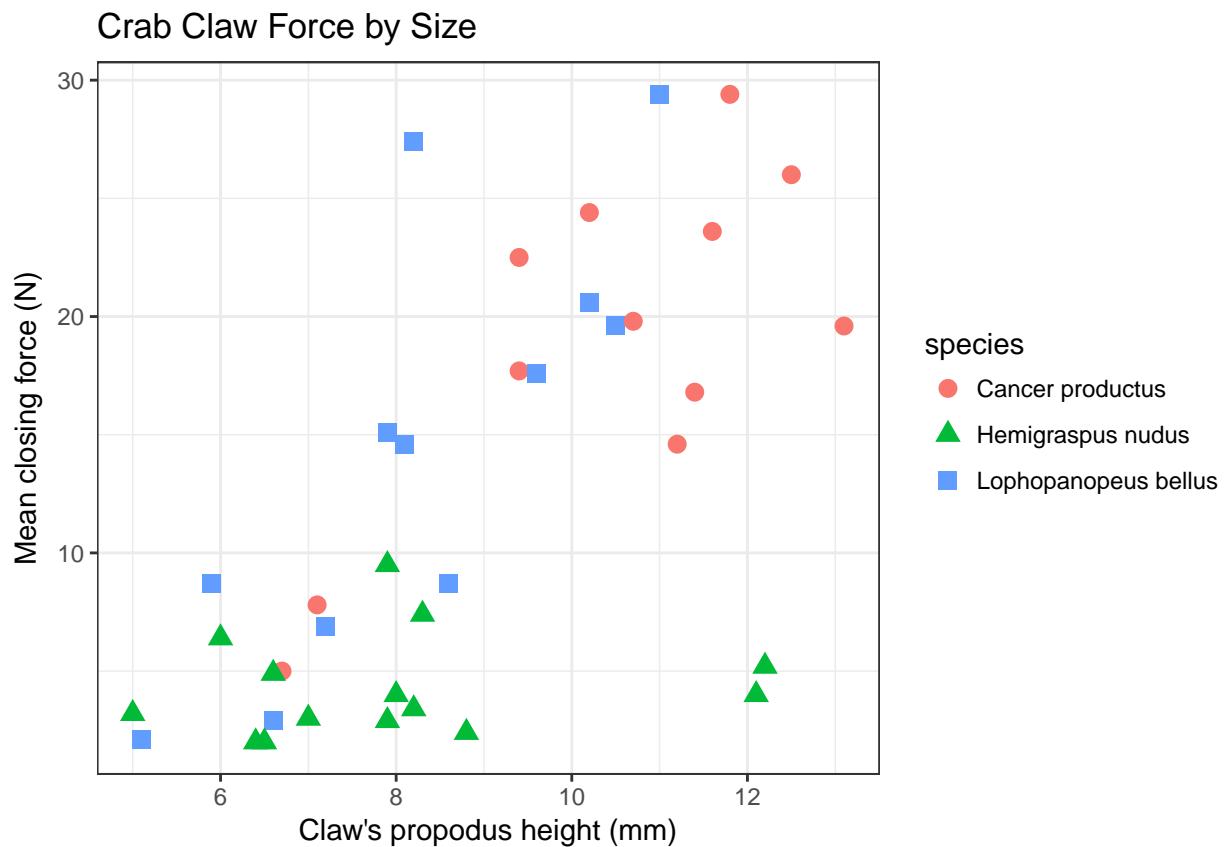
```
# A tibble: 3 x 4
  species      n `median(force)` `median(height)`
  <fct>     <int>        <dbl>        <dbl>
1 Cancer productus    12         19.7       10.95
2 Hemigrapsus nudus   14          3.7        7.90
3 Lophopanopeus bellus 12         14.8       8.15
```

## 12.1 Association of Size and Force

Suppose we want to describe force on the basis of height, across all 38 crabs. We'll add titles and identify the three species of crab, using shape and color.

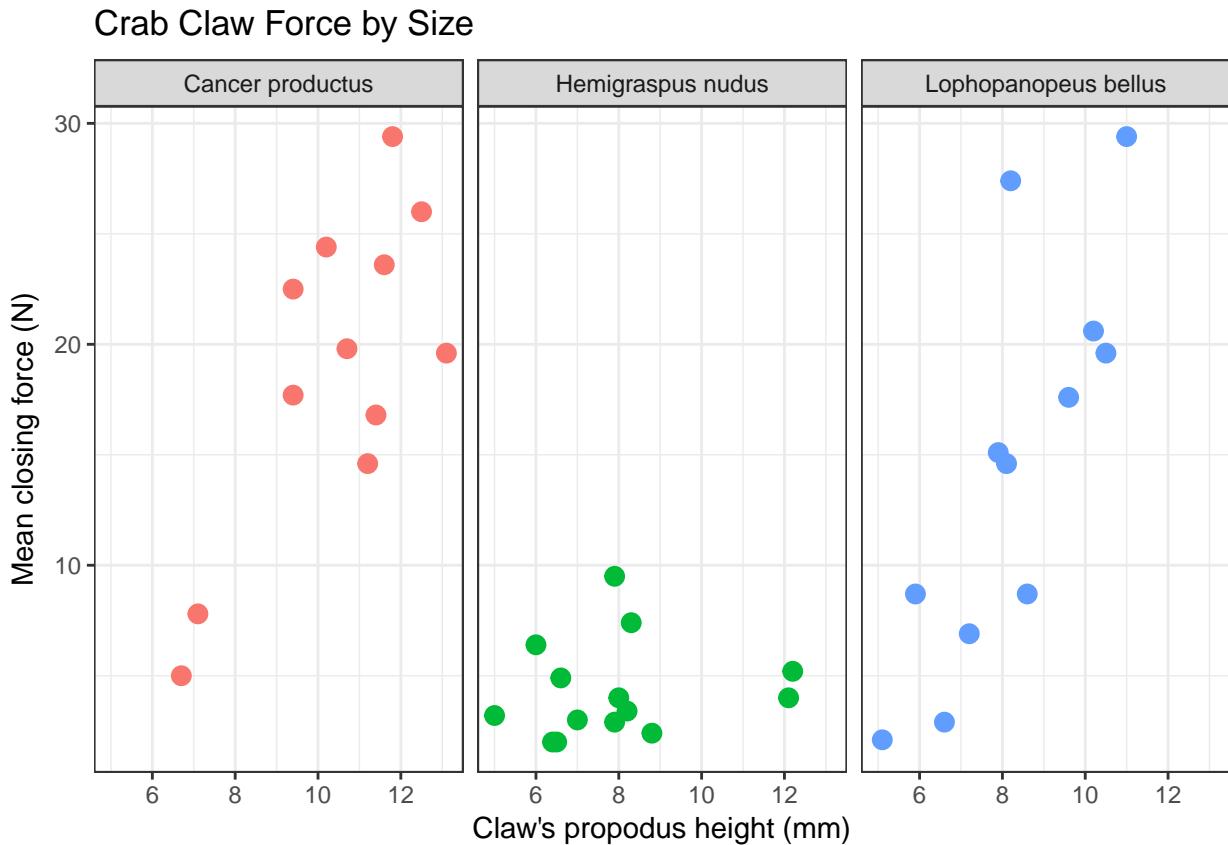
```
ggplot(crabs, aes(x = height, y = force, color = species, shape = species)) +
  geom_point(size = 3) +
  labs(title = "Crab Claw Force by Size",
```

```
x = "Claw's propodus height (mm)", y = "Mean closing force (N)" +
theme_bw()
```



A faceted plot for each species really highlights the difference in force between the *Hemigrapsus nudus* and the other two species of crab.

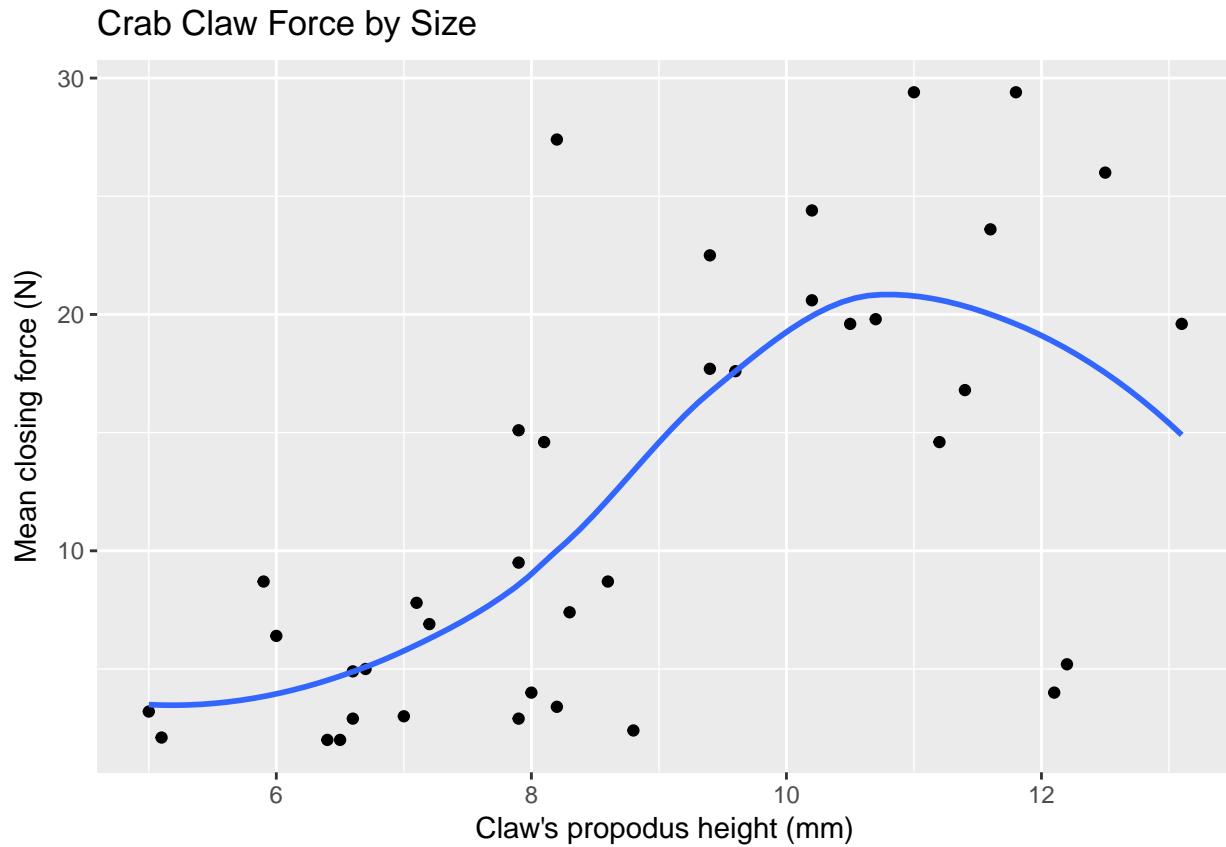
```
ggplot(crabs, aes(x = height, y = force, color = species)) +
  geom_point(size = 3) +
  facet_wrap(~ species) +
  guides(color = FALSE) +
  labs(title = "Crab Claw Force by Size",
       x = "Claw's propodus height (mm)", y = "Mean closing force (N)") +
  theme_bw()
```



## 12.2 The loess smooth

We can obtain a smoothed curve (using several different approaches) to summarize the pattern presented by the data in any scatterplot. For instance, we might build such a plot for the complete set of 38 crabs, adding in a non-linear smooth function (called a loess smooth.)

```
ggplot(crabs, aes(x = height, y = force)) +
  geom_point() +
  geom_smooth(method = "loess", se = FALSE) +
  labs(title = "Crab Claw Force by Size",
       x = "Claw's propodus height (mm)", y = "Mean closing force (N)")
```

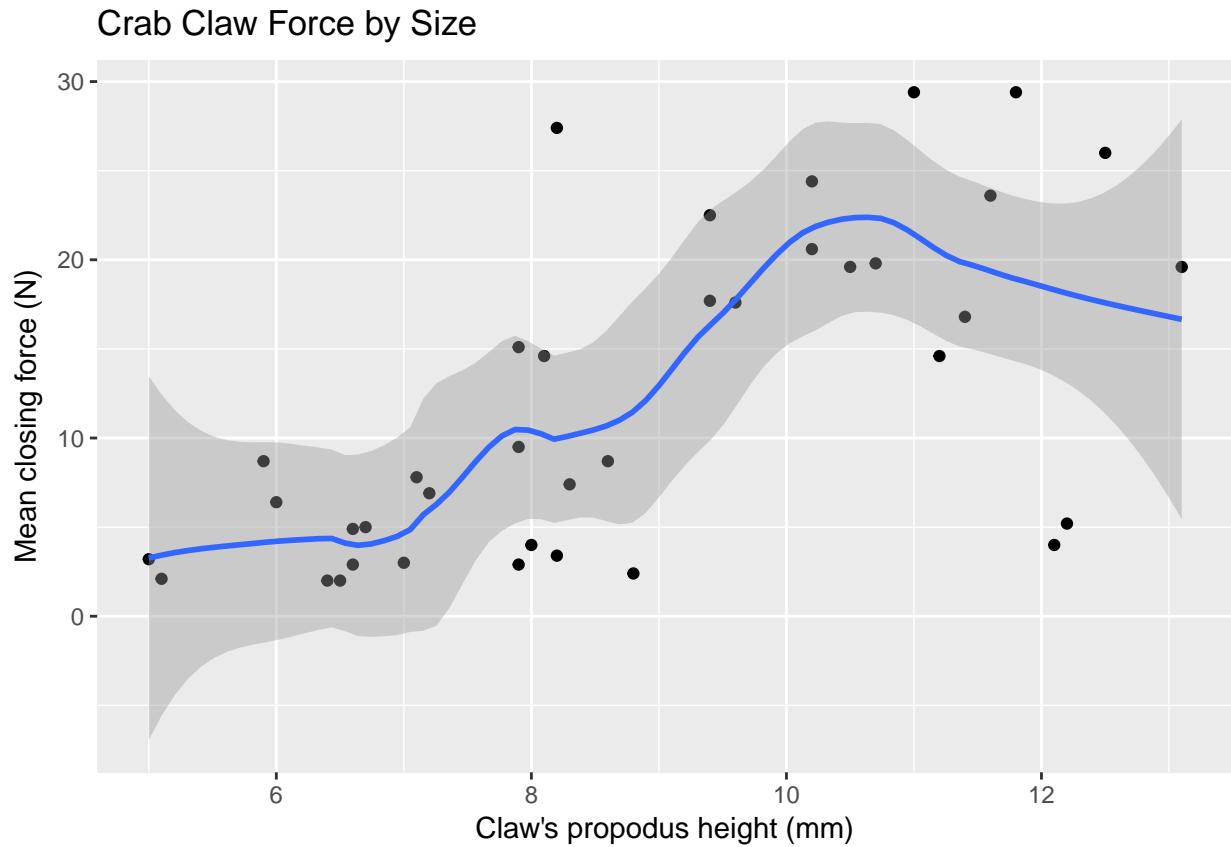


A **loess smooth** is a method of fitting a local polynomial regression model that R uses as its generic smooth for scatterplots with fewer than 1000 observations. Think of the loess as a way of fitting a curve to data by tracking (at point  $x$ ) the points within a neighborhood of point  $x$ , with more emphasis given to points near  $x$ . It can be adjusted by tweaking two specific parameters, in particular:

- a `span` parameter (defaults to 0.75) which is also called  $\alpha$  in the literature, that controls the degree of smoothing (essentially, how larger the neighborhood should be), and
- a `degree` parameter (defaults to 2) which specifies the degree of polynomial to be used. Normally, this is either 1 or 2 - more complex functions are rarely needed for simple scatterplot smoothing.

In addition to the curve, smoothing procedures can also provide confidence intervals around their main fitted line. Consider the following plot, which adjusts the span and also adds in the confidence intervals.

```
ggplot(crabs, aes(x = height, y = force)) +
  geom_point() +
  geom_smooth(method = "loess", span = 0.5, se = TRUE) +
  labs(title = "Crab Claw Force by Size",
       x = "Claw's propodus height (mm)", y = "Mean closing force (N)")
```

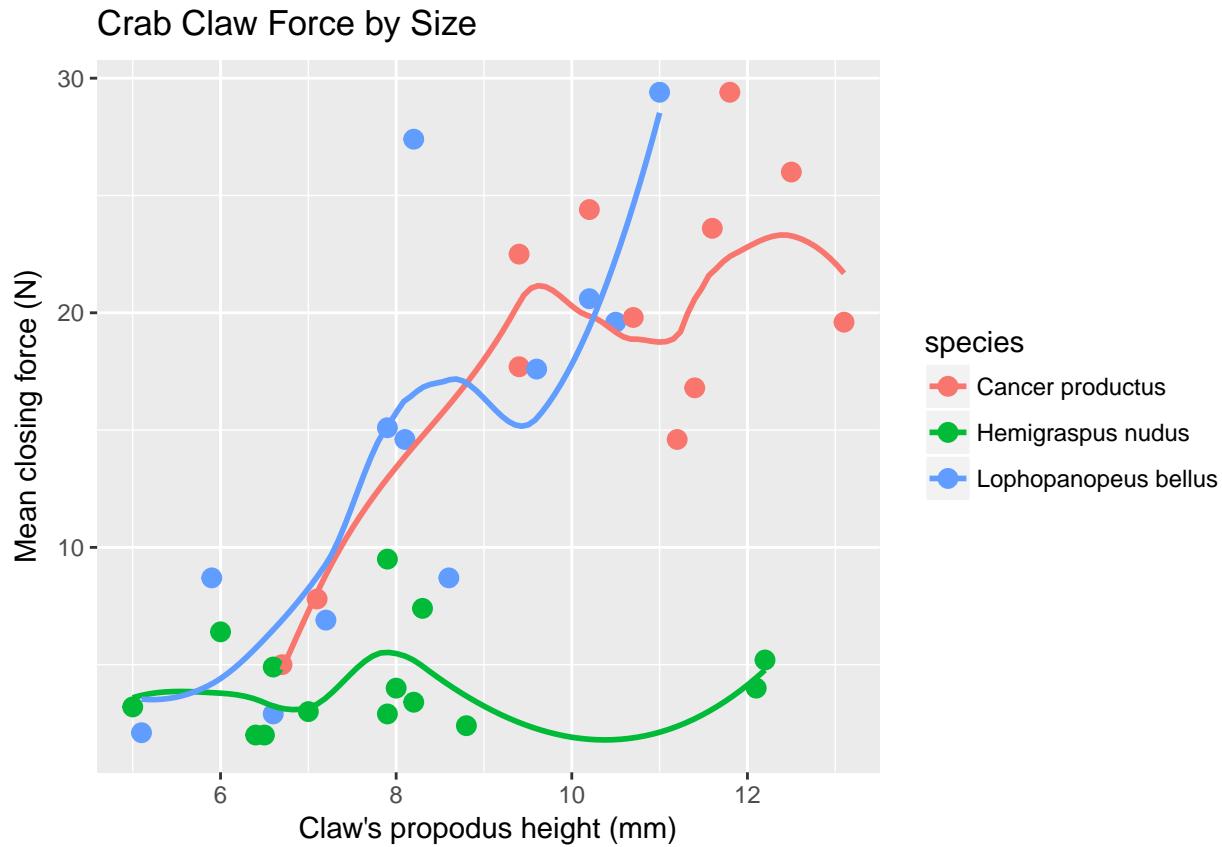


By reducing the size of the span, our resulting picture shows a much less smooth function that we generated previously.

### 12.2.1 Smoothing within Species

We can, of course, produce the plot above with separate smooths for each of the three species of crab.

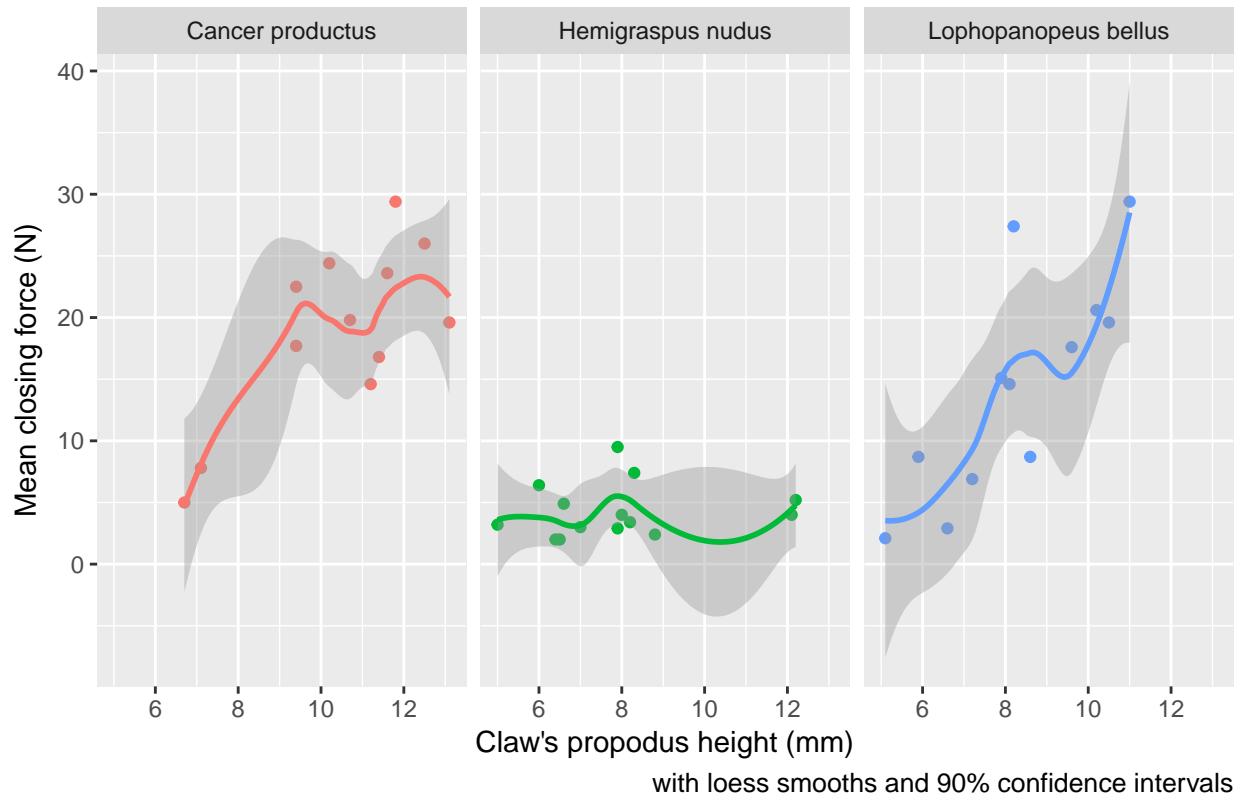
```
ggplot(crabs, aes(x = height, y = force, group = species, color = species)) +
  geom_point(size = 3) +
  geom_smooth(method = "loess", se = FALSE) +
  labs(title = "Crab Claw Force by Size",
       x = "Claw's propodus height (mm)", y = "Mean closing force (N)")
```



If we want to add in the confidence intervals (here I'll show them at 90% rather than the default of 95%) then this plot should be faceted. Note that by default, what is displayed when `se = TRUE` are 95% prediction intervals - the `level` function in `stat_smooth` [which can be used in place of `geom_smooth`] is used here to change the coverage percentage from 95% to 90%.

```
ggplot(crabs, aes(x = height, y = force, group = species, color = species)) +
  geom_point() +
  stat_smooth(method = "loess", level = 0.90, se = TRUE) +
  guides(color = FALSE) +
  labs(title = "Crab Claw Force by Size",
       caption = "with loess smooths and 90% confidence intervals",
       x = "Claw's propodus height (mm)", y = "Mean closing force (N)") +
  facet_wrap(~ species)
```

## Crab Claw Force by Size



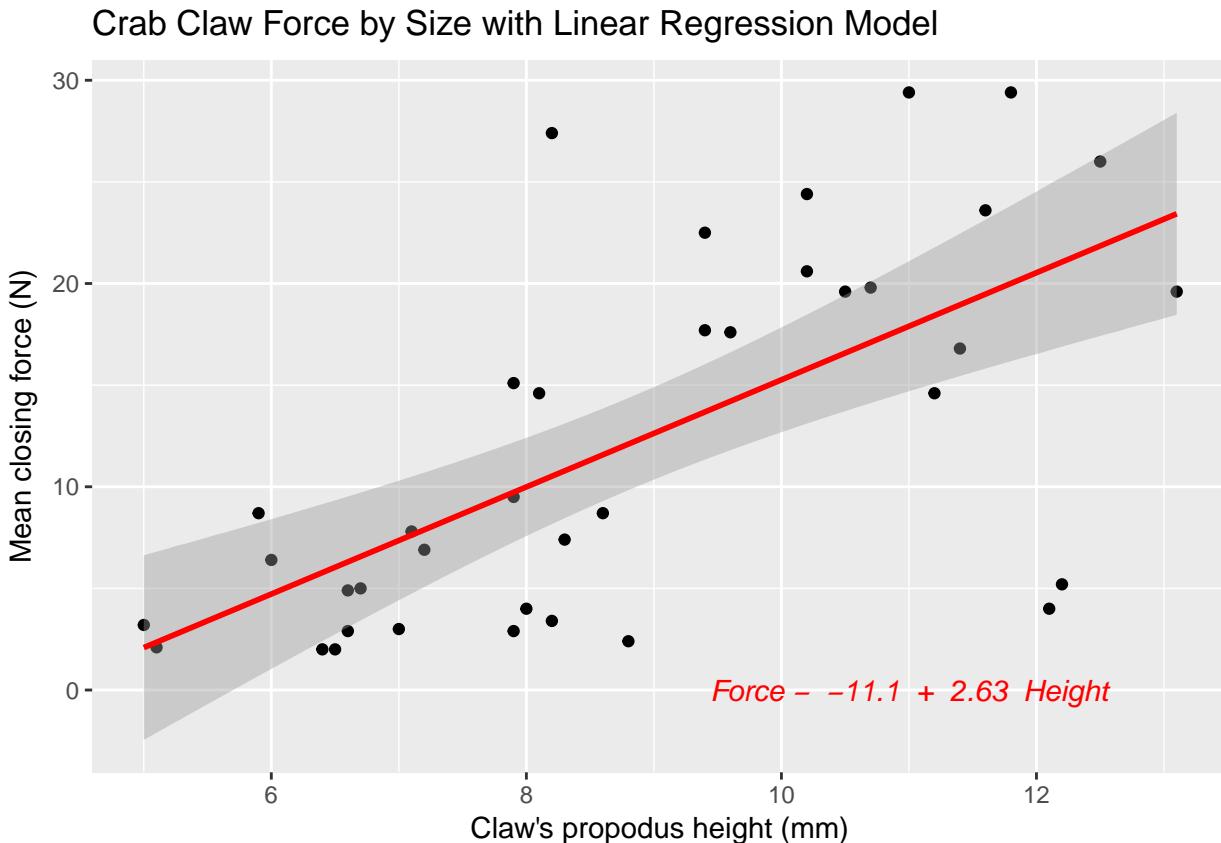
More on these and other confidence intervals later, especially in part B.

## 12.3 Fitting a Linear Regression Model

Suppose we plan to use a simple (least squares) linear regression model to describe force as a function of height. Is a least squares model likely to be an effective choice here?

The plot below shows the regression line predicting closing force as a function of propodus height. Here we annotate the plot to show the actual fitted regression line, which required fitting it with the `lm` statement prior to developing the graph.

```
mod <- lm(force ~ height, data = crabs)
```



```
rm(mod)
```

The **lm** function, again, specifies the linear model we fit to predict force using height. Here's the summary.

```
summary(lm(force ~ height, data = crabs))
```

Call:

```
lm(formula = force ~ height, data = crabs)
```

Residuals:

Min	1Q	Median	3Q	Max
-16.794	-3.811	-0.239	4.144	16.881

Coefficients:

	Estimate	Std. Error	t value	Pr(> t )
(Intercept)	-11.087	4.622	-2.40	0.022 *
height	2.635	0.509	5.18	8.7e-06 ***

---

Signif. codes: 0 '\*\*\*' 0.001 '\*\*' 0.01 '\*' 0.05 '.' 0.1 ' ' 1

Residual standard error: 6.89 on 36 degrees of freedom

Multiple R-squared: 0.427, Adjusted R-squared: 0.411

F-statistic: 26.8 on 1 and 36 DF, p-value: 8.73e-06

Again, the key things to realize are:

- The outcome variable in this model is **force**, and the predictor variable is **height**.

- The straight line model for these data fitted by least squares is force =  $-11.1 + 2.63 \text{ height}$ .
- The slope of height is positive, which indicates that as height increases, we expect that force will also increase. Specifically, we expect that for every additional mm of height, the force will increase by 2.63 Newtons.
- The multiple R-squared (squared correlation coefficient) is 0.427, which implies that 42.7% of the variation in force is explained using this linear model with height. It also implies that the Pearson correlation between force and height is the square root of 0.427, or 0.653.

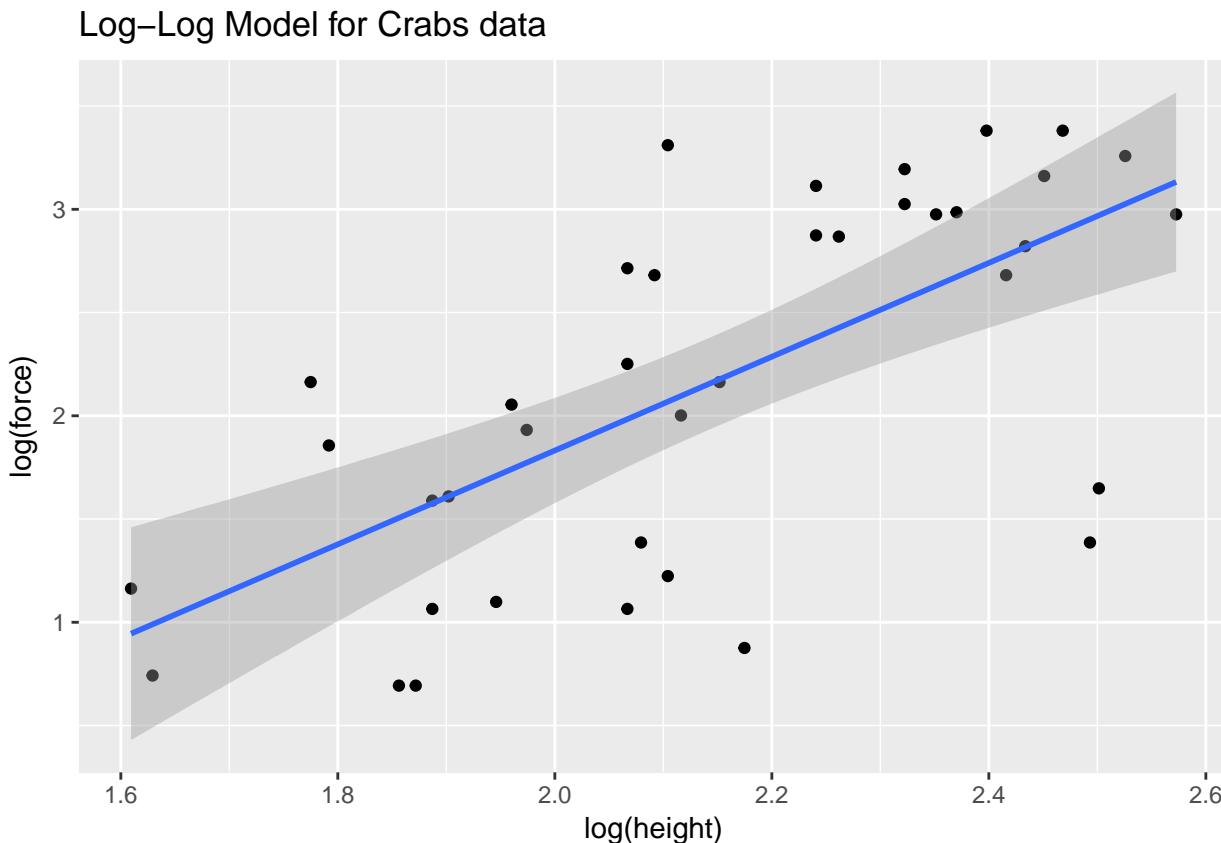
## 12.4 Is a Linear Model Appropriate?

The zoology (at least as described in Ramsey and Schafer (2002)) suggests that the actual nature of the relationship would be represented by a log-log relationship, where the log of force is predicted by the log of height.

This log-log model is an appropriate model when we think that percentage increases in X (height, here) lead to constant percentage increases in Y (here, force).

To see the log-log model in action, we plot the log of force against the log of height. We could use either base 10 (`log10` in R) or natural (`log` in R) logarithms.

```
ggplot(crabs, aes(x = log(height), y = log(force))) +
  geom_point() +
  geom_smooth(method = "lm") +
  labs(title = "Log-Log Model for Crabs data")
```



The correlations between the raw force and height and between their logarithms turn out to be quite similar,

and because the log transformation is monotone in these data, there's actually no change at all in the Spearman correlations.

Correlation of	Pearson r	Spearman r
force and height	0.653	0.657
log(force) and log(height)	0.662	0.657

### 12.4.1 The log-log model

```
crab_loglog <- lm(log(force) ~ log(height), data = crabs)

summary(crab_loglog)

Call:
lm(formula = log(force) ~ log(height), data = crabs)

Residuals:
    Min      1Q  Median      3Q     Max 
-1.566 -0.445  0.188  0.480  1.242 

Coefficients:
            Estimate Std. Error t value Pr(>|t|)    
(Intercept) -2.710     0.925   -2.93   0.0059 **  
log(height)  2.271     0.428    5.30   6e-06 ***  
---
Signif. codes:  0 '***' 0.001 '**' 0.01 '*' 0.05 '.' 0.1 ' ' 1

Residual standard error: 0.675 on 36 degrees of freedom
Multiple R-squared:  0.438, Adjusted R-squared:  0.423 
F-statistic: 28.1 on 1 and 36 DF,  p-value: 5.96e-06
```

Our regression equation is  $\log(\text{force}) = -2.71 + 2.27 \log(\text{height})$ .

So, for example, if we found a crab with propodus height = 10 mm, our prediction for that crab's claw force (in Newtons) based on this log-log model would be...

- $\log(\text{force}) = -2.71 + 2.27 \log(10)$
- $\log(\text{force}) = -2.71 + 2.27 \times 2.303$
- $\log(\text{force}) = 2.519$
- and so predicted force =  $\exp(2.519) = 12.417$  Newtons, which, naturally, we would round to 12.4 Newtons to match the data set's level of precision.

### 12.4.2 How does this compare to our original linear model?

```
crab_linear <- lm(force ~ height, data = crabs)

summary(crab_linear)
```

```
Call:
lm(formula = force ~ height, data = crabs)
```

Residuals:

Min	1Q	Median	3Q	Max
-16.794	-3.811	-0.239	4.144	16.881

Coefficients:

	Estimate	Std. Error	t value	Pr(> t )
(Intercept)	-11.087	4.622	-2.40	0.022 *
height	2.635	0.509	5.18	8.7e-06 ***
---				
Signif. codes:	0 '***'	0.001 '**'	0.01 '*'	0.05 '.'
	0.1 ''	1		

Residual standard error: 6.89 on 36 degrees of freedom

Multiple R-squared: 0.427, Adjusted R-squared: 0.411

F-statistic: 26.8 on 1 and 36 DF, p-value: 8.73e-06

The linear regression equation is force = -11.1 + 2.63 height.

So, for example, if we found a crab with propodus height = 10 mm, our prediction for that crab's claw force (in Newtons) based on this linear model would be...

- force = -11.087 + 2.635 x 10
- force = -11.087 + 26.348
- so predicted force = 15.261, which we would round to 15.3 Newtons.

So, it looks like the two models give meaningfully different predictions.

## 12.5 Making Predictions with a Model

A simpler way to get predictions for a new value like height = 10 mm from our models is available.

```
predict(crab_linear, data.frame(height = 10), interval = "prediction")
fit lwr upr
1 15.3 1.05 29.5
```

We'd interpret this result as saying that the linear model's predicted force associated with a single new crab claw with propodus height 10 mm is 15.3 Newtons, and that a 95% prediction interval for the true value of such a force for such a claw is between 1.0 and 29.5 Newtons. More on prediction intervals later.

### 12.5.1 Predictions After a Transformation

We can also get predictions from the log-log model.

```
predict(crab_loglog, data.frame(height = 10), interval = "prediction")
fit lwr upr
1 2.52 1.13 3.91
```

Of course, this prediction is of the `log(force)` for such a crab claw. To get the prediction in terms of simple force, we'd need to back out of the logarithm, by exponentiating our point estimate and the prediction interval endpoints.

```
exp(predict(crab_loglog, data.frame(height = 10), interval = "prediction"))
fit lwr upr
1 12.4 3.08 50
```

We'd interpret this result as saying that the log-log model's predicted force associated with a single new crab claw with propodus height 10 mm is 12.4 Newtons, and that a 95% prediction interval for the true value of such a force for such a claw is between 3.1 and 50.0 Newtons.

### 12.5.2 Comparing Model Predictions

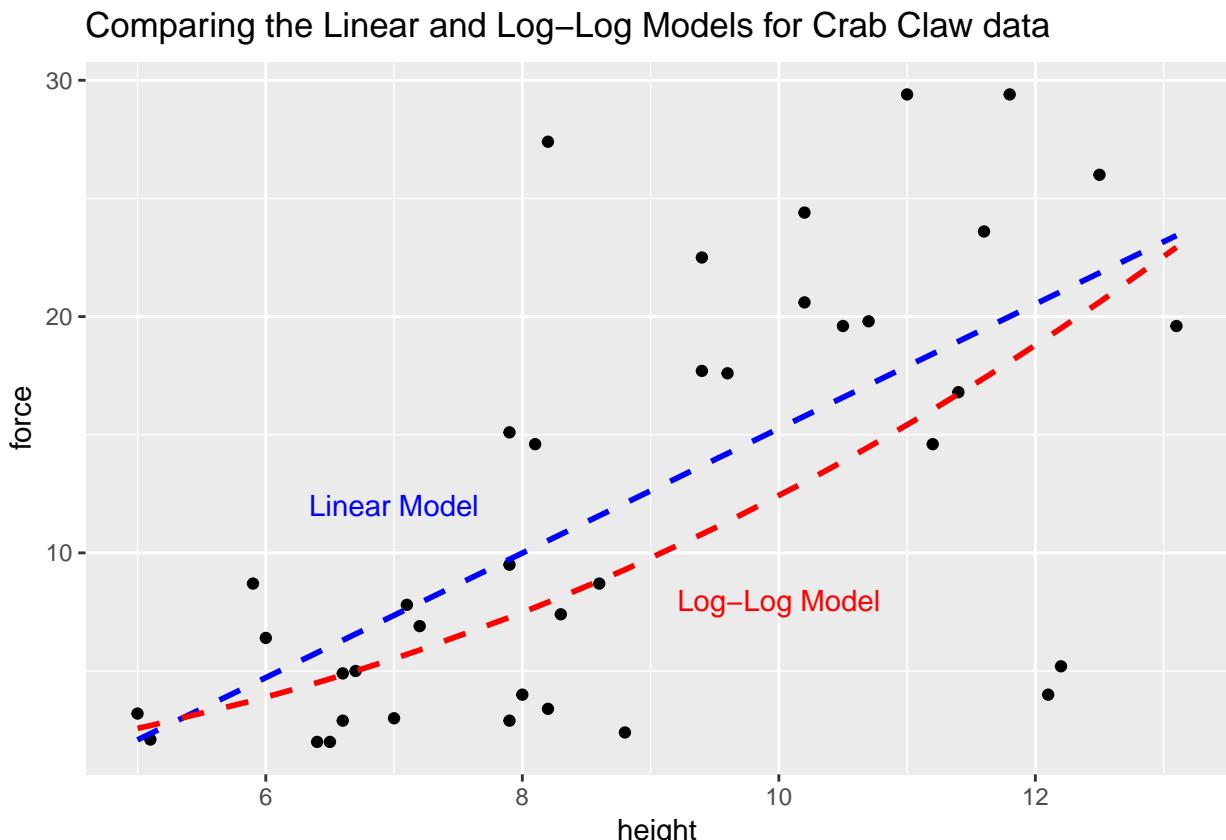
Suppose we wish to build a plot of force vs height with a straight line for the linear model's predictions, and a new curve for the log-log model's predictions, so that we can compare and contrast the implications of the two models on a common scale. The `predict` function, when not given a new data frame, will use the existing predictor values that are in our `crabs` data. Such predictions are often called fitted values.

To put the two sets of predictions on the same scale despite the differing outcomes in the two models, we'll exponentiate the results of the log-log model, and build a little data frame containing the heights and the predicted forces from that model.

```
loglogdat <- data.frame(height = crabs$height, force = exp(predict(crab_loglog)))
```

Now, we're ready to use the `geom_smooth` approach to plot the linear fit, and `geom_line` (which also fits curves) to display the log-log fit.

```
ggplot(crabs, aes(x = height, y = force)) +
  geom_point() +
  geom_smooth(method = "lm", se = FALSE, col="blue", linetype = 2) +
  geom_line(data = loglogdat, col = "red", linetype = 2, size = 1) +
  annotate("text", 7, 12, label = "Linear Model", col = "blue") +
  annotate("text", 10, 8, label = "Log-Log Model", col = "red") +
  labs(title = "Comparing the Linear and Log-Log Models for Crab Claw data")
```



Based on these 38 crabs, we see some modest differences between the predictions of the two models, with the log-log model predicting generally lower closing force for a given propodus height than would be predicted by a linear model.

```
rm(loglogdat, crab_linear, crab_loglog)
```

# Chapter 13

## The Western Collaborative Group Study

### 13.1 The Western Collaborative Group Study (`wcgs`) data set

Vittinghoff et al. (2012) explore data from the Western Collaborative Group Study (WCGS) in great detail<sup>1</sup>. We'll touch lightly on some key issues in this Chapter.

```
wcgs <- read.csv("data/wcgs.csv") %>%tbl_df  
  
wcgs  
  
# A tibble: 3,154 x 22  
  id    age   agec height weight lnwght wghtcat   bmi    sbp lnsbp   dbp  
  <int> <int> <fctr> <int> <int> <dbl> <fctr> <dbl> <int> <dbl> <int>  
1 2343    50 46-50     67    200   5.30 170-200  31.3    132  4.88    90  
2 3656    51 51-55     73    192   5.26 170-200  25.3    120  4.79    74  
3 3526    59 56-60     70    200   5.30 170-200  28.7    158  5.06    94  
4 22057   51 51-55     69    150   5.01 140-170  22.1    126  4.84    80  
5 12927   44 41-45     71    160   5.08 140-170  22.3    126  4.84    80  
6 16029   47 46-50     64    158   5.06 140-170  27.1    116  4.75    76  
7 3894    40 35-40     70    162   5.09 140-170  23.2    122  4.80    78  
8 11389   41 41-45     70    160   5.08 140-170  23.0    130  4.87    84  
9 12681   50 46-50     71    195   5.27 170-200  27.2    112  4.72    70  
10 10005   43 41-45    68    187   5.23 170-200  28.4    120  4.79    80  
# ... with 3,144 more rows, and 11 more variables: chol <int>,  
#   behpat <fctr>, dibpat <fctr>, smoke <fctr>, ncigs <int>, arcus <int>,  
#   chd69 <fctr>, typchd69 <int>, time169 <int>, t1 <dbl>, uni <dbl>
```

Here, we have 3154 rows (subjects) and 22 columns (variables).

#### 13.1.1 Structure of `wcgs`

We can specify the (sometimes terrible) variable names, through the `names` function, or we can add other elements of the structure, so that we can identify elements of particular interest.

<sup>1</sup>For more on the WCGS, you might look at <http://www.epi.umn.edu/cvdepi/study-synopsis/western-collaborative-group-study/>

```
str(wcgs)
```

```
Classes 'tbl_df', 'tbl' and 'data.frame': 3154 obs. of 22 variables:
 $ id      : int  2343 3656 3526 22057 12927 16029 3894 11389 12681 10005 ...
 $ age     : int  50 51 59 51 44 47 40 41 50 43 ...
 $ agec    : Factor w/ 5 levels "35-40","41-45",...: 3 4 5 4 2 3 1 2 3 2 ...
 $ height   : int  67 73 70 69 71 64 70 70 71 68 ...
 $ weight   : int  200 192 200 150 160 158 162 160 195 187 ...
 $ lnwght   : num  5.3 5.26 5.3 5.01 5.08 ...
 $ wghtcat : Factor w/ 4 levels "< 140","> 200",...: 4 4 4 3 3 3 3 3 4 4 ...
 $ bmi     : num  31.3 25.3 28.7 22.1 22.3 ...
 $ sbp     : int  132 120 158 126 126 116 122 130 112 120 ...
 $ lnsbp   : num  4.88 4.79 5.06 4.84 4.84 ...
 $ dbp     : int  90 74 94 80 80 76 78 84 70 80 ...
 $ chol    : int  249 194 258 173 214 206 190 212 130 233 ...
 $ behpat  : Factor w/ 4 levels "A1","A2","B3",...: 1 1 1 1 1 1 1 1 1 1 ...
 $ dibpat  : Factor w/ 2 levels "Type A","Type B": 1 1 1 1 1 1 1 1 1 1 ...
 $ smoke   : Factor w/ 2 levels "No","Yes": 2 2 1 1 1 2 1 2 1 2 ...
 $ ncigs   : int  25 25 0 0 0 80 0 25 0 25 ...
 $ arcus   : int  1 0 1 1 0 0 0 0 1 0 ...
 $ chd69   : Factor w/ 2 levels "No","Yes": 1 1 1 1 1 1 1 1 1 1 ...
 $ typchd69: int  0 0 0 0 0 0 0 0 0 0 ...
 $ time169 : int  1367 2991 2960 3069 3081 2114 2929 3010 3104 2861 ...
 $ t1      : num  -1.63 -4.06 0.64 1.12 2.43 ...
 $ uni     : num  0.486 0.186 0.728 0.624 0.379 ...
```

### 13.1.2 Codebook for wcgs

This table was lovingly hand-crafted, and involved a lot of typing. We'll look for better ways in 432.

Name	Stored As	Type	Details (units, levels, etc.)
id	integer	(nominal)	ID #, nominal and uninteresting
age	integer	quantitative	age, in years - no decimal places
agec	factor (5)	(ordinal)	age: 35-40, 41-45, 46-50, 51-55, 56-60
height	integer	quantitative	height, in inches
weight	integer	quantitative	weight, in pounds
lnwght	number	quantitative	natural logarithm of weight
wghtcat	factor (4)	(ordinal)	wt: < 140, 140-170, 170-200, > 200
bmi	number	quantitative	body-mass index: $703 * \text{weight in lb} / (\text{height in in})^2$
sbp	integer	quantitative	systolic blood pressure, in mm Hg
lnsbp	number	quantitative	natural logarithm of sbp
dbp	integer	quantitative	diastolic blood pressure, mm Hg
chol	integer	quantitative	total cholesterol, mg/dL
behpat	factor (4)	(nominal)	behavioral pattern: A1, A2, B3 or B4
dibpat	factor (2)	(binary)	behavioral pattern: A or B
smoke	factor (2)	(binary)	cigarette smoker: Yes or No
ncigs	integer	quantitative	number of cigarettes smoked per day
arcus	integer	(nominal)	arcus senilis present (1) or absent (0)
chd69	factor (2)	(binary)	CHD event: Yes or No
typchd69	integer	(4 levels)	event: 0 = no CHD, 1 = MI or SD, 2 = silent MI, 3 = angina

Name	Stored As	Type	Details (units, levels, etc.)
time169	integer	quantitative	follow-up time in days
t1	number	quantitative	heavy-tailed (random draws)
uni	number	quantitative	light-tailed (random draws)

### 13.1.3 Quick Summary

```
summary(wcgs)
```

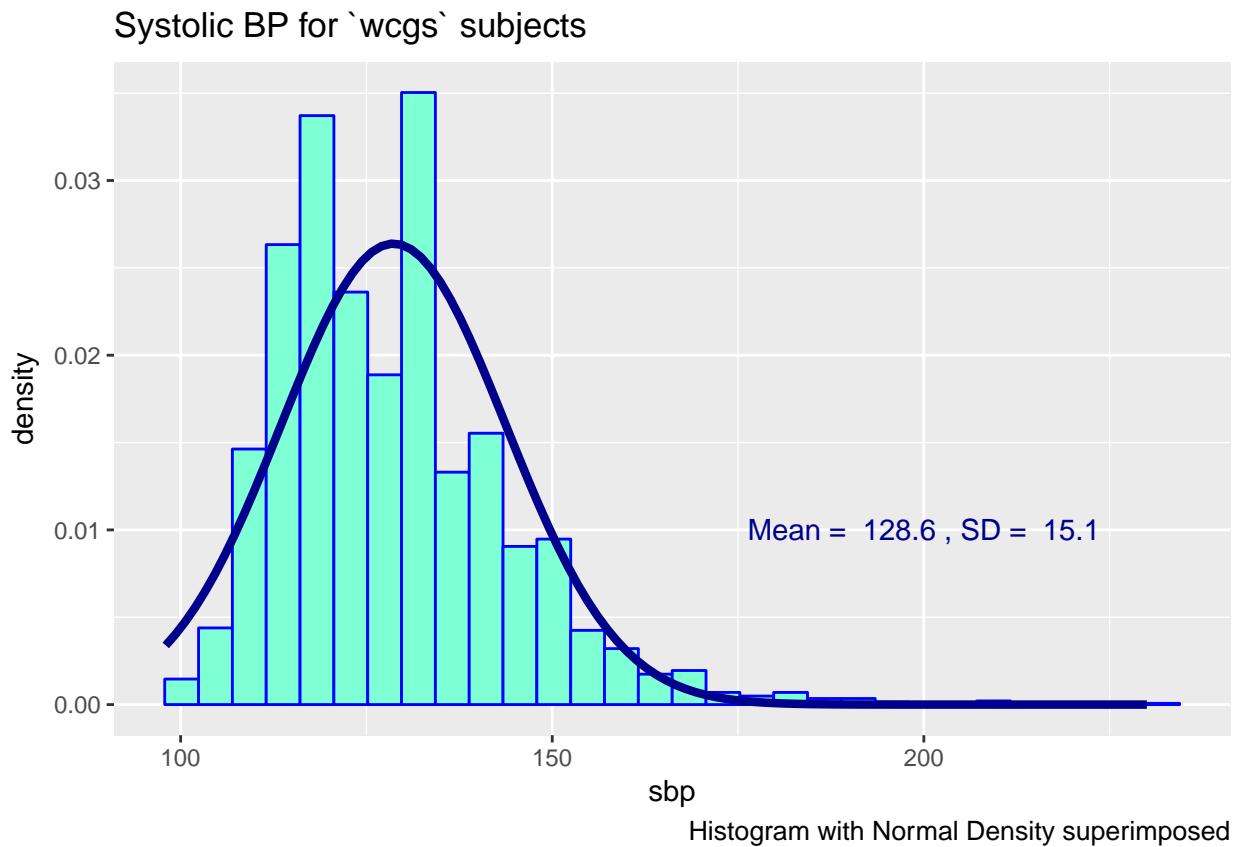
id	age	agec	height	weight
Min. : 2001	Min. :39.0	35-40: 543	Min. :60.0	Min. : 78
1st Qu.: 3741	1st Qu.:42.0	41-45:1091	1st Qu.:68.0	1st Qu.:155
Median :11406	Median :45.0	46-50: 750	Median :70.0	Median :170
Mean :10478	Mean :46.3	51-55: 528	Mean :69.8	Mean :170
3rd Qu.:13115	3rd Qu.:50.0	56-60: 242	3rd Qu.:72.0	3rd Qu.:182
Max. :22101	Max. :59.0		Max. :78.0	Max. :320
lnwght	wghtcat	bmi	sbp	lnsbp
Min. :4.36	< 140 : 232	Min. :11.2	Min. : 98	Min. :4.58
1st Qu.:5.04	> 200 : 213	1st Qu.:23.0	1st Qu.:120	1st Qu.:4.79
Median :5.14	140-170:1538	Median :24.4	Median :126	Median :4.84
Mean :5.13	170-200:1171	Mean :24.5	Mean :129	Mean :4.85
3rd Qu.:5.20		3rd Qu.:25.8	3rd Qu.:136	3rd Qu.:4.91
Max. :5.77		Max. :38.9	Max. :230	Max. :5.44
dbp	chol	behpap	dibpat	smoke
Min. : 58	Min. :103	A1: 264	Type A:1589	No :1652
1st Qu.: 76	1st Qu.:197	A2:1325	Type B:1565	Yes:1502
Median : 80	Median :223	B3:1216		
Mean : 82	Mean :226	B4: 349		
3rd Qu.: 86	3rd Qu.:253			
Max. :150	Max. :645			
	NA's :12			
ncigs	arcus	chd69	typchd69	time169
Min. : 0.0	Min. :0.000	No :2897	Min. :0.000	Min. : 18
1st Qu.: 0.0	1st Qu.:0.000	Yes: 257	1st Qu.:0.000	1st Qu.:2842
Median : 0.0	Median :0.000		Median :0.000	Median :2942
Mean :11.6	Mean :0.299		Mean :0.136	Mean :2684
3rd Qu.:20.0	3rd Qu.:1.000		3rd Qu.:0.000	3rd Qu.:3037
Max. :99.0	Max. :1.000		Max. :3.000	Max. :3430
	NA's :2			
t1	uni			
Min. :-47.4	Min. :0.001			
1st Qu.: -1.0	1st Qu.:0.257			
Median : 0.0	Median :0.516			
Mean : 0.0	Mean :0.505			
3rd Qu.: 1.0	3rd Qu.:0.756			
Max. : 47.0	Max. :0.999			
NA's :39				

For a more detailed description, we might consider `Hmisc::describe`, `psych::describe`, `mosaic::favstats`, etc.

## 13.2 Are the SBPs Normally Distributed?

Consider the question of whether the distribution of the systolic blood pressure results is well-approximated by the Normal.

```
ggplot(wcgs, aes(x = sbp)) +
  geom_histogram(aes(y = ..density..),
                 bins = 30, fill = "aquamarine", col="blue") +
  stat_function(fun = dnorm, lwd = 1.5, col = "darkblue",
                args = list(mean = mean(wcgs$sbp), sd = sd(wcgs$sbp))) +
  annotate("text", x = 200, y = 0.01, col = "darkblue",
           label = paste("Mean = ", round(mean(wcgs$sbp),1),
                         ", SD = ", round(sd(wcgs$sbp),1))) +
  labs(title = "Systolic BP for `wcgs` subjects",
       caption = "Histogram with Normal Density superimposed")
```



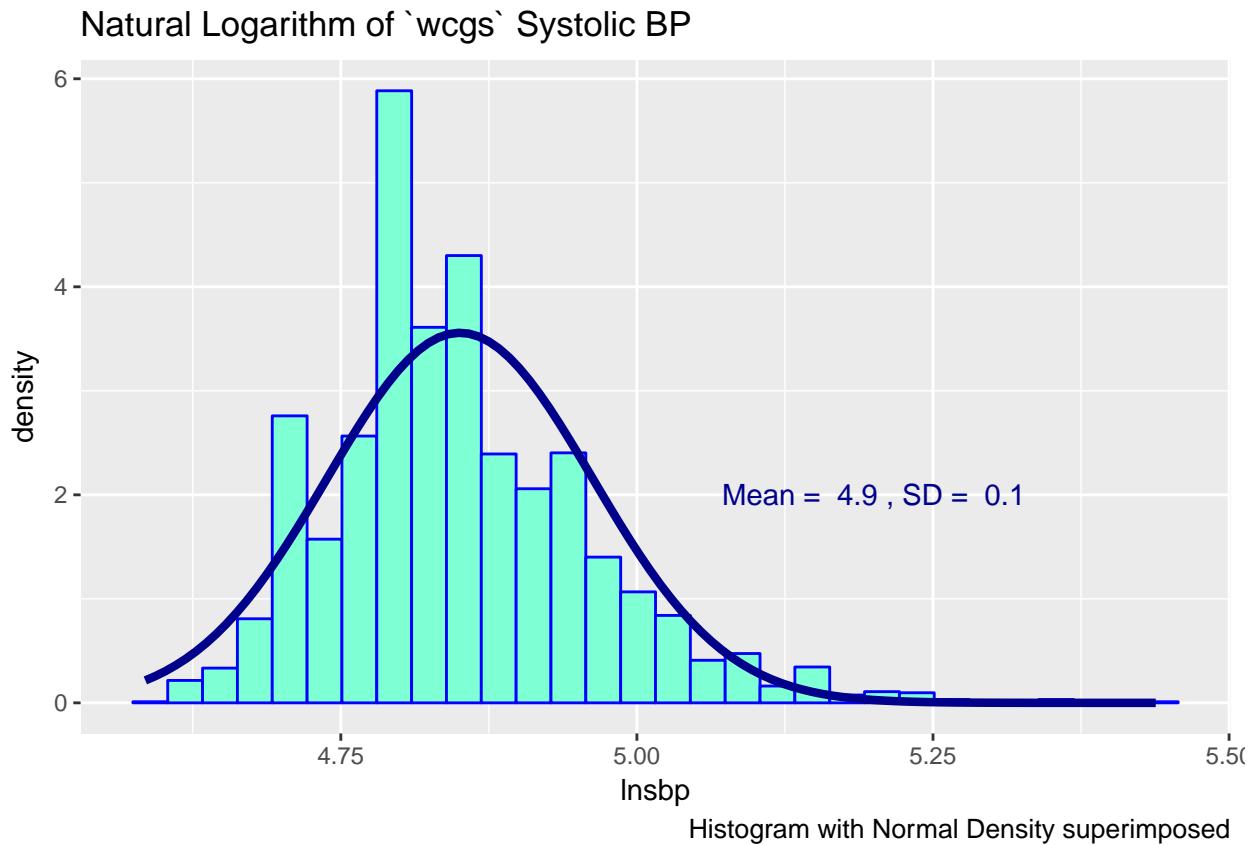
Since the data contain both `sbp` and `lnsbp` (its natural logarithm), let's compare them. Note that in preparing the graph, we'll need to change the location for the text annotation.

```
ggplot(wcgs, aes(x = lnsbp)) +
  geom_histogram(aes(y = ..density..),
                 bins = 30, fill = "aquamarine", col="blue") +
  stat_function(fun = dnorm, lwd = 1.5, col = "darkblue",
                args = list(mean = mean(wcgs$lnsbp),
                           sd = sd(wcgs$lnsbp))) +
  annotate("text", x = 5.2, y = 2, col = "darkblue",
           label = paste("Mean = ", round(mean(wcgs$lnsbp),1),
```

```

    ", SD = " , round(sd(wcgs$lnsbp),1))) +
  labs(title = "Natural Logarithm of `wcgs` Systolic BP",
       caption = "Histogram with Normal Density superimposed")

```



We can also look at Normal Q-Q plots, for instance...

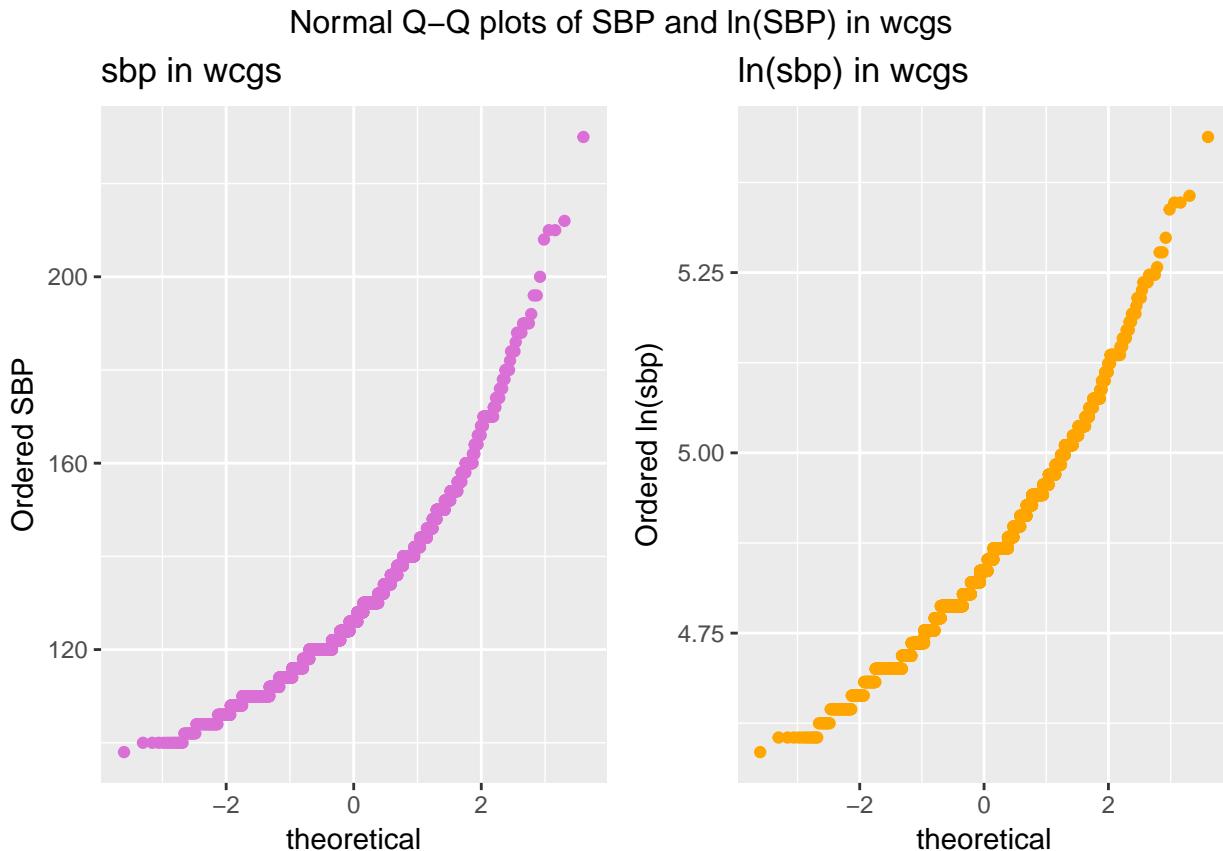
```

p1 <- ggplot(wcgs, aes(sample = sbp)) +
  geom_point(stat="qq", color = "orchid") +
  labs(y = "Ordered SBP", title = "sbp in wcgs")

p2 <- ggplot(wcgs, aes(sample = lnsbp)) +
  geom_point(stat="qq", color = "orange") +
  labs(y = "Ordered ln(sbp)", title = "ln(sbp) in wcgs")

gridExtra::grid.arrange(p1, p2, ncol=2, top ="Normal Q-Q plots of SBP and ln(SBP) in wcgs")

```



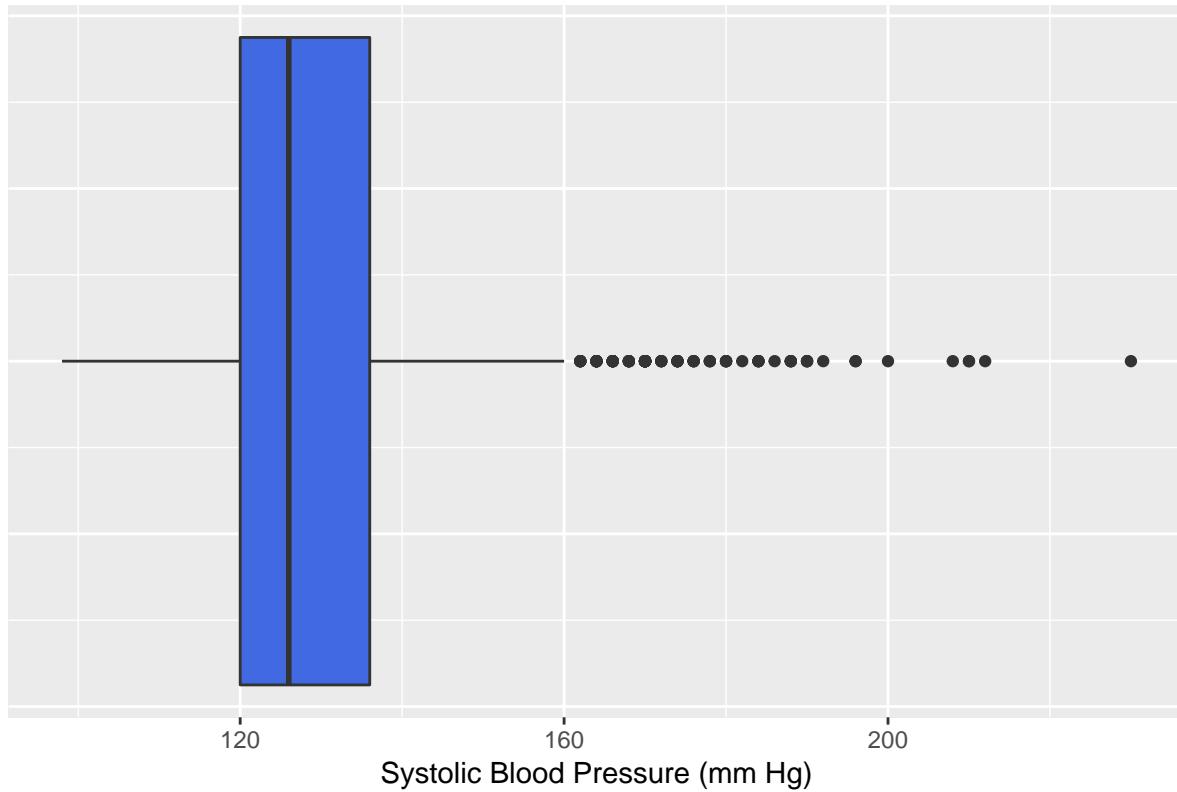
There's at best a small improvement from `sbp` to `ln(sbp)` in terms of approximation by a Normal distribution.

### 13.3 Describing Outlying Values with Z Scores

It looks like there's an outlier (or a series of them) in the SBP data.

```
ggplot(wcgs, aes(x = 1, y = sbp)) +
  geom_boxplot(fill = "royalblue") +
  labs(title = "Boxplot of SBP in `wcgs` data",
       y = "Systolic Blood Pressure (mm Hg)",
       x = "") +
  theme(axis.text.y = element_blank(),
        axis.ticks.y = element_blank()) +
  coord_flip()
```

Boxplot of SBP in `wcgs` data



```
Hmisc::describe(wcgs$sbp)
```

wcgs\$sbp							
n	missing	distinct	Info	Mean	Gmd	.05	.10
3154	0	62	0.996	128.6	16.25	110	112
.25	.50	.75	.90	.95			
120	126	136	148	156			

lowest : 98 100 102 104 106, highest: 200 208 210 212 230

The maximum value here is 230, and is clearly the most extreme value in the data set. One way to gauge this is to describe that observation's **Z score**, the number of standard deviations away from the mean that the observation falls. Here, the maximum value, 230 is 6.71 standard deviations above the mean, and thus has a Z score of 6.7.

A negative Z score would indicate a point below the mean, while a positive Z score indicates, as we've seen, a point above the mean. The minimum systolic blood pressure, 98 is 2.03 standard deviations *below* the mean, so it has a Z score of -2.

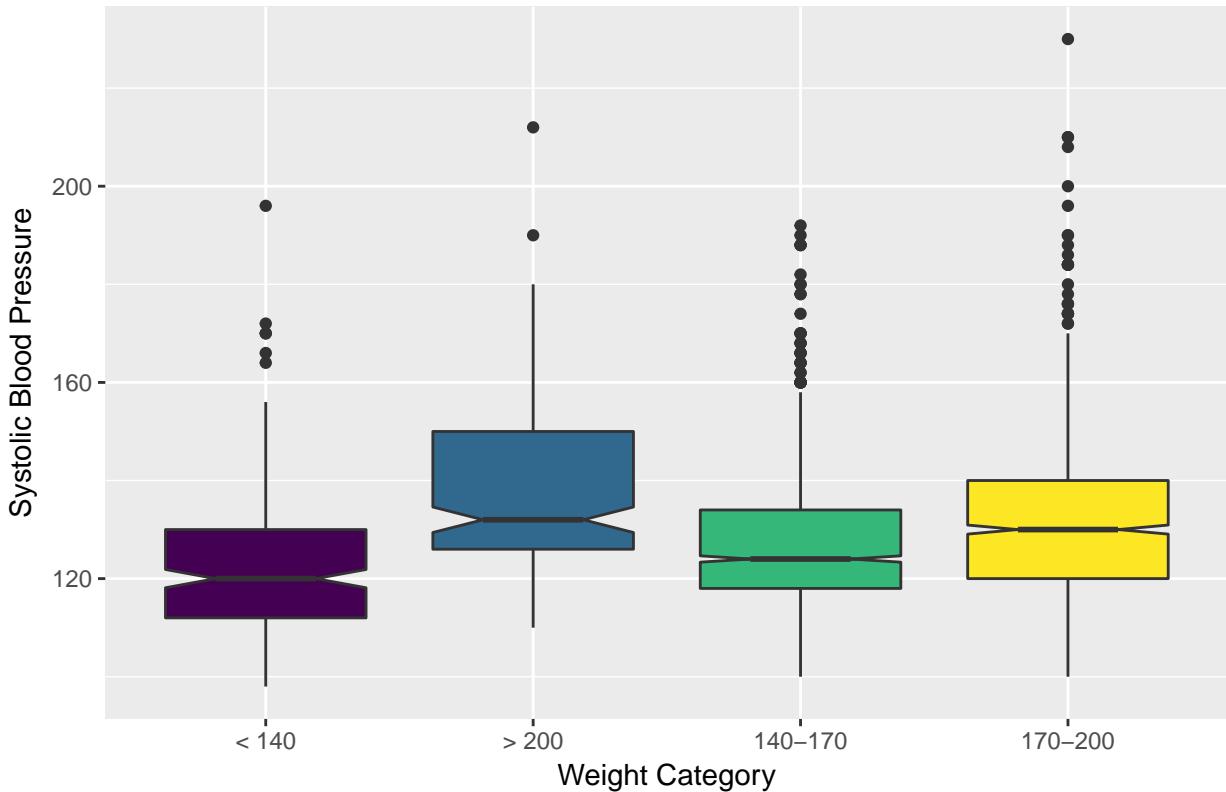
Recall that the Empirical Rule suggests that if a variable follows a Normal distribution, it would have approximately 95% of its observations falling inside a Z score of (-2, 2), and 99.74% falling inside a Z score range of (-3, 3). Do the systolic blood pressures appear Normally distributed?

## 13.4 Does Weight Category Relate to SBP?

The data are collected into four groups based on the subject's weight (in pounds).

```
ggplot(wcgs, aes(x = wghtcat, y = sbp, fill = wghtcat)) +
  geom_boxplot(notch = TRUE) +
  scale_fill_viridis(discrete=TRUE) +
  guides(fill = FALSE) +
  labs(title = "Boxplot of Systolic BP by Weight Category in WCGS",
       x = "Weight Category", y = "Systolic Blood Pressure")
```

Boxplot of Systolic BP by Weight Category in WCGS



## 13.5 Re-Leveling a Factor

Well, that's not so good. We really want those weight categories (the *levels*) to be ordered more sensibly.

```
table(wcgs$wghtcat)
```

```
< 140    > 200  140-170  170-200
 232      213    1538     1171
```

Like all *factor* variables in R, the categories are specified as levels.

```
levels(wcgs$wghtcat)
```

```
[1] "< 140"    "> 200"    "140-170"  "170-200"
```

We want to change the order of the levels in a new version of this factor variable so they make sense. There are multiple ways to do this, but I prefer the `fct_relevel` function from the `forcats` package. Which order is more appropriate?

```
table(fct_relevel(wcgs$wghtcat, "< 140", "140-170", "170-200", "> 200"), wcgs$wghtcat)
```

	< 140	> 200	140-170	170-200
< 140	232	0	0	0
140-170	0	0	1538	0
170-200	0	0	0	1171
> 200	0	213	0	0

I'll add a new variable to the `wcgs` data called `weight_f` that relevels the `wghtcat` data.

```
wcgs <- wcgs %>%
  mutate(weight_f = fct_relevel(wghtcat, "< 140", "140-170", "170-200", "> 200"))

table(wcgs$weight_f)
```

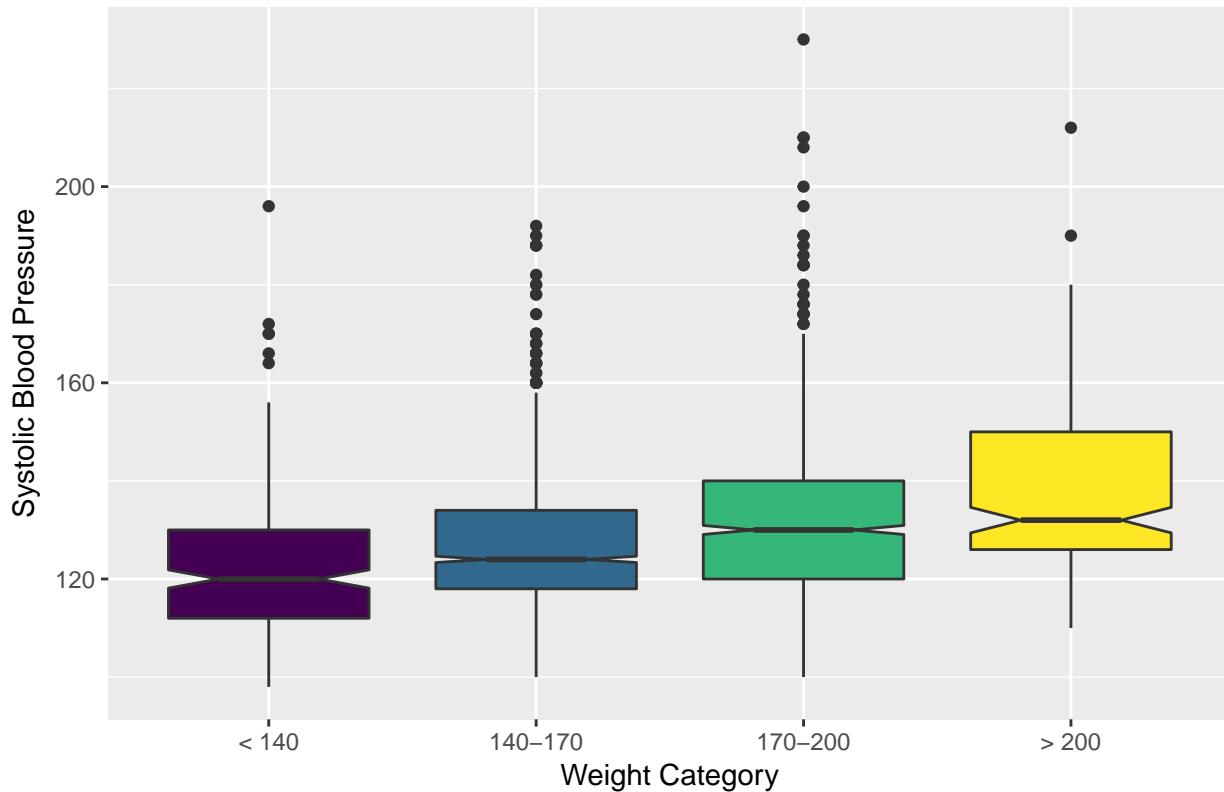
< 140	140-170	170-200	> 200
232	1538	1171	213

For more on the `forcats` package, check out Gromelund and Wickham (2017), especially the Section on Factors.

### 13.5.1 SBP by Weight Category

```
ggplot(wcgs, aes(x = weight_f, y = sbp, fill = weight_f)) +
  geom_boxplot(notch = TRUE) +
  scale_fill_viridis(discrete=TRUE) +
  guides(fill = FALSE) +
  labs(title = "Systolic Blood Pressure by Reordered Weight Category in WCGS",
       x = "Weight Category", y = "Systolic Blood Pressure")
```

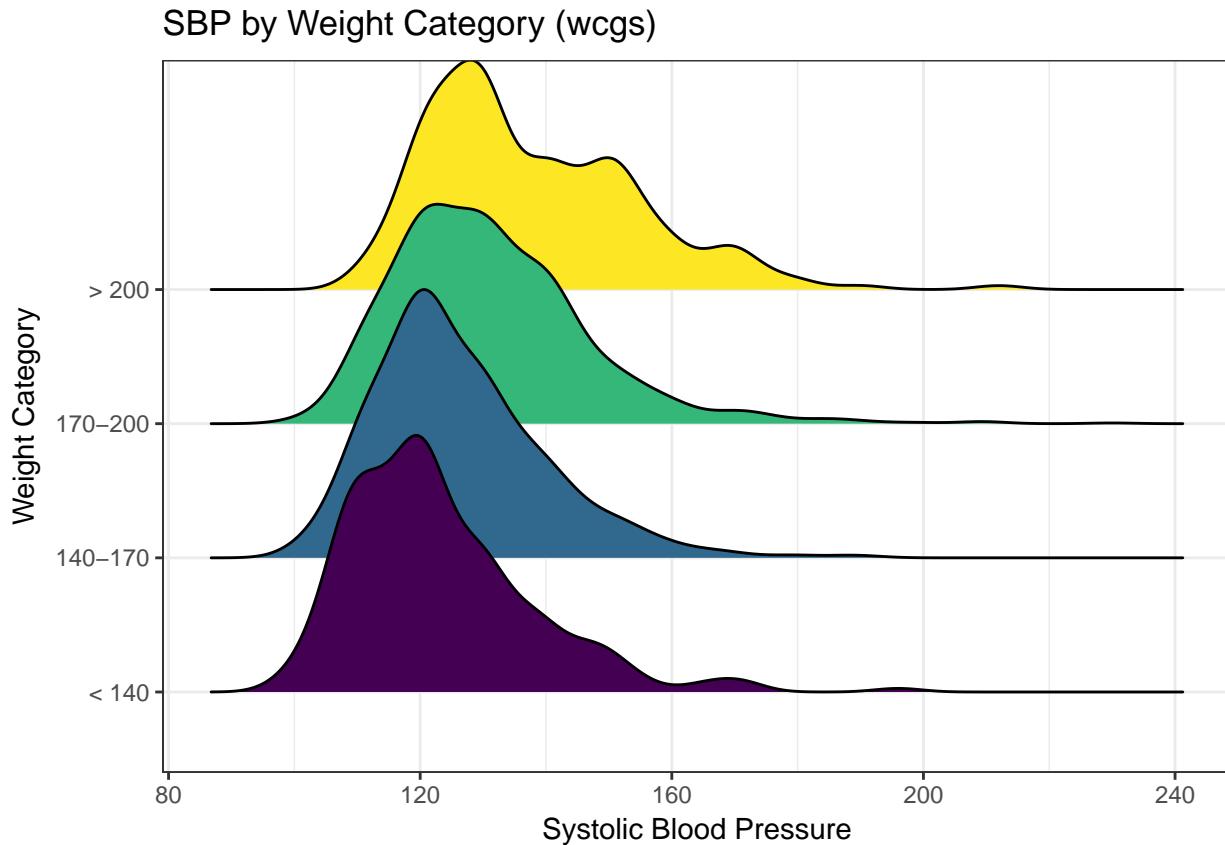
### Systolic Blood Pressure by Reordered Weight Category in WCGS



We might see some details well with a **ridgeline plot**, too.

```
wcgs %>%
  ggplot(aes(x = sbp, y = weight_f, fill = weight_f, height = ..density..)) +
  ggridges::geom_density_ridges(scale = 2) +
  scale_fill_viridis(discrete = TRUE) +
  guides(fill = FALSE) +
  labs(title = "SBP by Weight Category (wcgs)",
       x = "Systolic Blood Pressure",
       y = "Weight Category") +
  theme_bw()
```

Picking joint bandwidth of 3.74



As the plots suggest, patients in the heavier groups generally had higher systolic blood pressures.

```
by(wcgs$sbp, wcgs$weight_f, mosaic::favstats)

wcgs$weight_f: < 140
  min   Q1 median   Q3 max mean   sd   n missing
  98 112     120 130 196 123 14.7 232      0

-----
wcgs$weight_f: 140-170
  min   Q1 median   Q3 max mean   sd   n missing
 100 118     124 134 192 126 13.7 1538      0

-----
wcgs$weight_f: 170-200
  min   Q1 median   Q3 max mean   sd   n missing
 100 120     130 140 230 131 15.6 1171      0

-----
wcgs$weight_f: > 200
  min   Q1 median   Q3 max mean   sd   n missing
 110 126     132 150 212 138 16.8 213      0
```

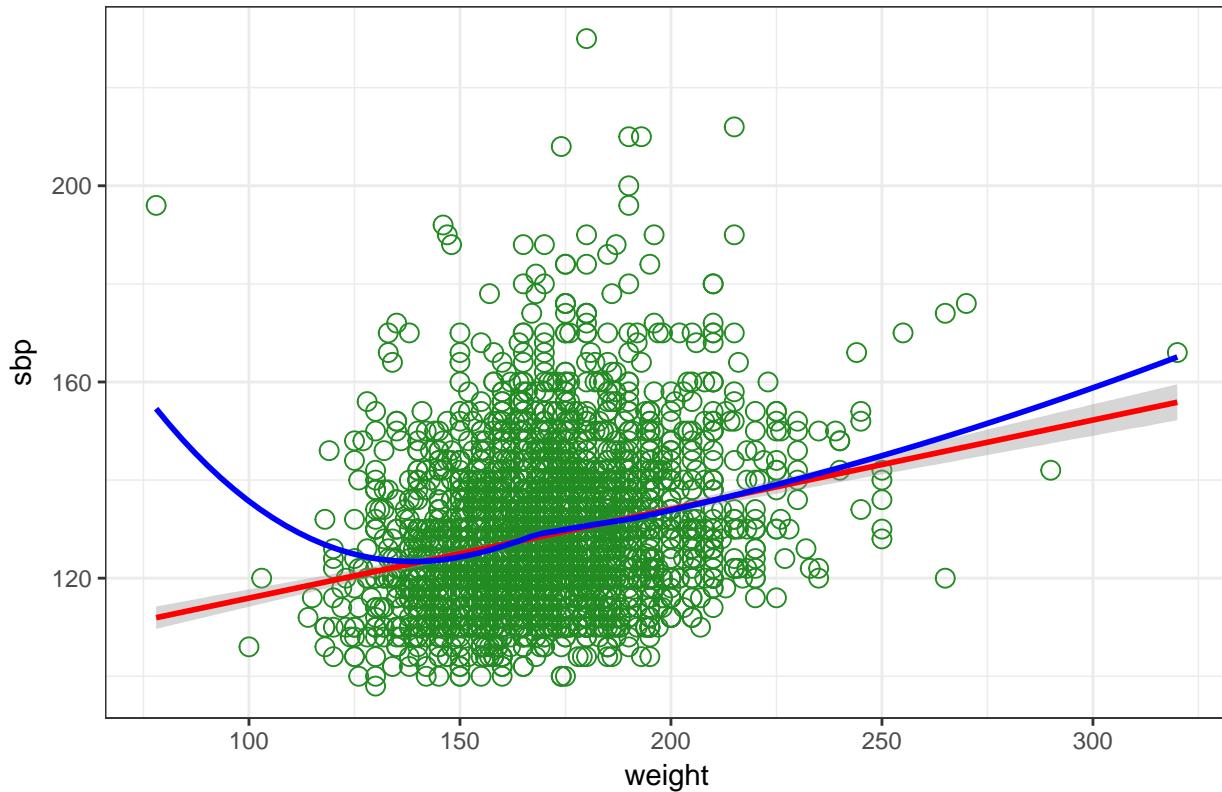
## 13.6 Are Weight and SBP Linked?

Let's build a scatter plot of SBP (Outcome) by Weight (Predictor), rather than breaking down into categories.

```
ggplot(wcgs, aes(x = weight, y = sbp)) +
  geom_point(size=3, shape=1, color="forestgreen") + ## default size = 2
```

```
stat_smooth(method=lm, color="red") + ## add se=FALSE to hide conf. interval
stat_smooth(method=loess, se=FALSE, color="blue") +
ggtitle("SBP vs. Weight in 3,154 WCGS Subjects") +
theme_bw()
```

SBP vs. Weight in 3,154 WCGS Subjects



- The mass of the data is hidden from us - showing 3154 points in one plot can produce little more than a blur where there are lots of points on top of each other.
- Here the least squares regression line (in red), and loess scatterplot smoother, (in blue) can help.

The relationship between systolic blood pressure and weight appears to be very close to linear, but of course there is considerable scatter around that generally linear relationship. It turns out that the Pearson correlation of these two variables is 0.253.

### 13.7 SBP and Weight by Arcus Senilis groups?

An issue of interest to us will be to assess whether the SBP-Weight relationship we see above is similar among subjects who have arcus senilis and those who do not.

Arcus senilis is an old age syndrome where there is a white, grey, or blue opaque ring in the corneal margin (peripheral corneal opacity), or white ring in front of the periphery of the iris. It is present at birth but then fades; however, it is quite commonly present in the elderly. It can also appear earlier in life as a result of hypercholesterolemia.

Wikipedia article on Arcus Senilis, retrieved 2017-08-15

Let's start with a quick look at the `arcus` data.

```
wcgs %>%
  select(arcus) %>%
  summary()
```

```
arcus
Min.    :0.000
1st Qu.:0.000
Median  :0.000
Mean    :0.299
3rd Qu.:1.000
Max.    :1.000
NA's    :2
```

We have 2 missing values, so we probably want to do something about that before plotting the data, and we may also want to create a factor variable with more meaningful labels than 1 (which means yes, arcus senilis is present) and 0 (which means no, it isn't.) We'll use the

```
wcgs <- wcgs %>%
  mutate(arcus_f = fct_recode(factor(arcus),
                               "Arcus senilis" = "1",
                               "No arcus senilis" = "0"),
         arcus_f = fct_relevel(arcus_f, "Arcus senilis"))

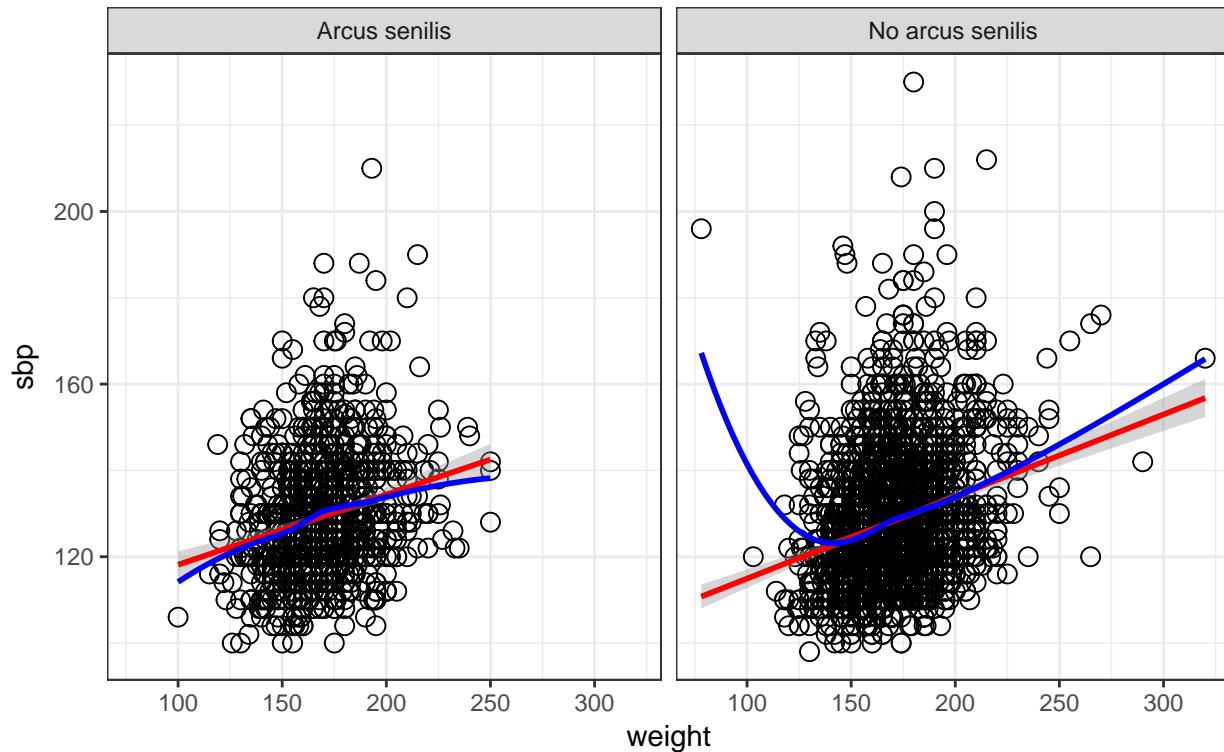
table(wcgs$arcus_f, wcgs$arcus, useNA = "ifany")
```

	0	1	<NA>
Arcus senilis	0	941	0
No arcus senilis	2211	0	0
<NA>	0	0	2

Let's build a version of the `wcgs` data that eliminates all missing data in the variables of immediate interest, and then plot the SBP-weight relationship in groups of patients with and without arcus senilis.

```
wcgs %>%
  filter(complete.cases(arcus_f, sbp, weight)) %>%
  ggplot(aes(x = weight, y = sbp, group = arcus_f)) +
  geom_point(size=3, shape = 1) +
  stat_smooth(method=lm, color="red") +
  stat_smooth(method=loess, se=FALSE, color="blue") +
  labs(title = "SBP vs. Weight by Arcus Senilis status",
       caption = "3,152 Western Collaborative Group Study subjects with known arcus senilis status") +
  facet_wrap(~ arcus_f) +
  theme_bw()
```

### SBP vs. Weight by Arcus Senilis status



### 13.8 Linear Model for SBP-Weight Relationship: subjects without Arcus Senilis

```
model.noarcus <-
  lm(sbp ~ weight, data = filter(wcgs, arcus == 0))

summary(model.noarcus)
```

Call:  
`lm(formula = sbp ~ weight, data = filter(wcgs, arcus == 0))`

Residuals:

Min	1Q	Median	3Q	Max
-29.01	-10.25	-2.45	7.55	99.85

Coefficients:

	Estimate	Std. Error	t value	Pr(> t )
(Intercept)	95.9219	2.5552	37.5	<2e-16 ***
weight	0.1902	0.0149	12.8	<2e-16 ***

---

Signif. codes: 0 '\*\*\*' 0.001 '\*\*' 0.01 '\*' 0.05 '.' 0.1 ' ' 1

```
Residual standard error: 14.8 on 2209 degrees of freedom
Multiple R-squared:  0.0687,    Adjusted R-squared:  0.0683
F-statistic: 163 on 1 and 2209 DF,  p-value: <2e-16
```

The linear model for the 2211 patients without Arcus Senilis has  $R^2 = 6.87\%$ .

- The regression equation is  $95.92 - 0.19 \text{ weight}$ , for those patients without Arcus Senilis.

## 13.9 Linear Model for SBP-Weight Relationship: subjects with Arcus Senilis

```
model.witharcus <-
  lm(sbp ~ weight, data = filter(wcgs, arcus == 1))

summary(model.witharcus)
```

```
Call:
lm(formula = sbp ~ weight, data = filter(wcgs, arcus == 1))
```

Residuals:

Min	1Q	Median	3Q	Max
-30.34	-9.64	-1.96	7.97	76.74

Coefficients:

	Estimate	Std. Error	t value	Pr(> t )							
(Intercept)	101.879	3.756	27.13	< 2e-16 ***							
weight	0.163	0.022	7.39	3.3e-13 ***							
---											
Signif. codes:	0	'***'	0.001	'**'	0.01	'*'	0.05	'. '	0.1	' '	1

```
Residual standard error: 14.2 on 939 degrees of freedom
Multiple R-squared:  0.0549,    Adjusted R-squared:  0.0539
F-statistic: 54.6 on 1 and 939 DF,  p-value: 3.29e-13
```

The linear model for the 941 patients with Arcus Senilis has  $R^2 = 5.49\%$ .

- The regression equation is  $101.88 - 0.163 \text{ weight}$ , for those patients with Arcus Senilis.

## 13.10 Including Arcus Status in the model

```
model3 <- lm(sbp ~ weight * arcus, data = filter(wcgs, !is.na(arcus)))

summary(model3)
```

```
Call:
lm(formula = sbp ~ weight * arcus, data = filter(wcgs, !is.na(arcus)))
```

Residuals:

Min	1Q	Median	3Q	Max
-30.34	-10.15	-2.35	7.67	99.85

Coefficients:

	Estimate	Std. Error	t value	Pr(> t )							
(Intercept)	95.9219	2.5244	38.00	<2e-16 ***							
weight	0.1902	0.0147	12.92	<2e-16 ***							
arcus	5.9566	4.6197	1.29	0.20							
weight:arcus	-0.0276	0.0270	-1.02	0.31							
---											
Signif. codes:	0	'***'	0.001	'**'	0.01	'*'	0.05	.	0.1	' '	1

Residual standard error: 14.6 on 3148 degrees of freedom

Multiple R-squared: 0.066, Adjusted R-squared: 0.0651

F-statistic: 74.1 on 3 and 3148 DF, p-value: <2e-16

The actual regression equation in this setting includes both weight, and an indicator variable (1 = yes, 0 = no) for arcus senilis status, and the product of weight and that 1/0 indicator.

- Note the use of the product term `weight*arcus` in the setup of the model to allow both the slope of weight and the intercept term in the model to change depending on arcus senilis status.
  - For a patient who has arcus, the regression equation is  $SBP = 95.92 - 0.19 \text{ weight} + 5.96 (1) - 0.028 \text{ weight} (1) = 101.88 + 0.162 \text{ weight}$ .
  - For a patient without arcus senilis, the regression equation is  $SBP = 95.92 - 0.19 \text{ weight} + 5.96 (0) - 0.028 \text{ weight} (0) = 95.92 - 0.19 \text{ weight}$ .

The linear model including the interaction of weight and arcus to predict sbp for the 3152 patients with known Arcus Senilis status has  $R^2 = 6.6\%$ .

## 13.11 Predictions from these Linear Models

What is our predicted SBP for a subject weighing 175 pounds?

How does that change if our subject weighs 200 pounds?

Recall that

- *Without* Arcus Senilis, linear model for  $SBP = 95.9 + 0.19 \times \text{weight}$
- *With* Arcus Senilis, linear model for  $SBP = 101.9 + 0.16 \times \text{weight}$

So the predictions for a 175 pound subject are:  $- 95.9 + 0.19 \times 175 = 129$  mm Hg without Arcus Senilis, and  $- 101.9 + 0.16 \times 175 = 130$  mm Hg with Arcus Senilis.

And thus, the predictions for a 200 pound subject are:  $- 95.9 + 0.19 \times 200 = 134$  mm Hg without Arcus Senilis, and  $- 101.9 + 0.16 \times 200 = 134.4$  mm Hg with Arcus Senilis.

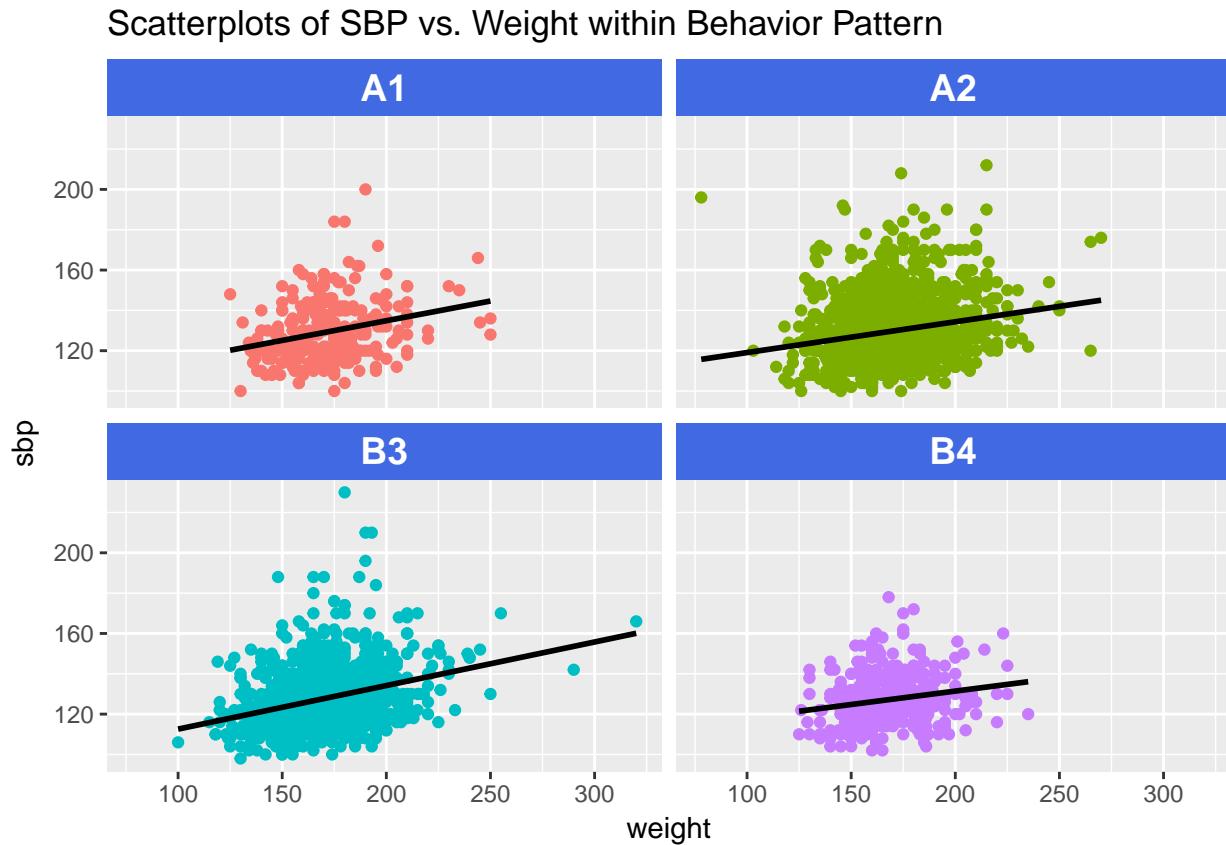
```
rm(model.noarcus, model.witharcus)
```

## 13.12 Scatterplots with Facets Across a Categorical Variable

We can use facets in `ggplot2` to show scatterplots across the levels of a categorical variable, like `behpat`.

```
ggplot(wcgs, aes(x = weight, y = sbp, col = behpat)) +
  geom_point() +
  facet_wrap(~ behpat) +
  geom_smooth(method = "lm", se = FALSE, col = "black") +
  guides(color = FALSE) +
```

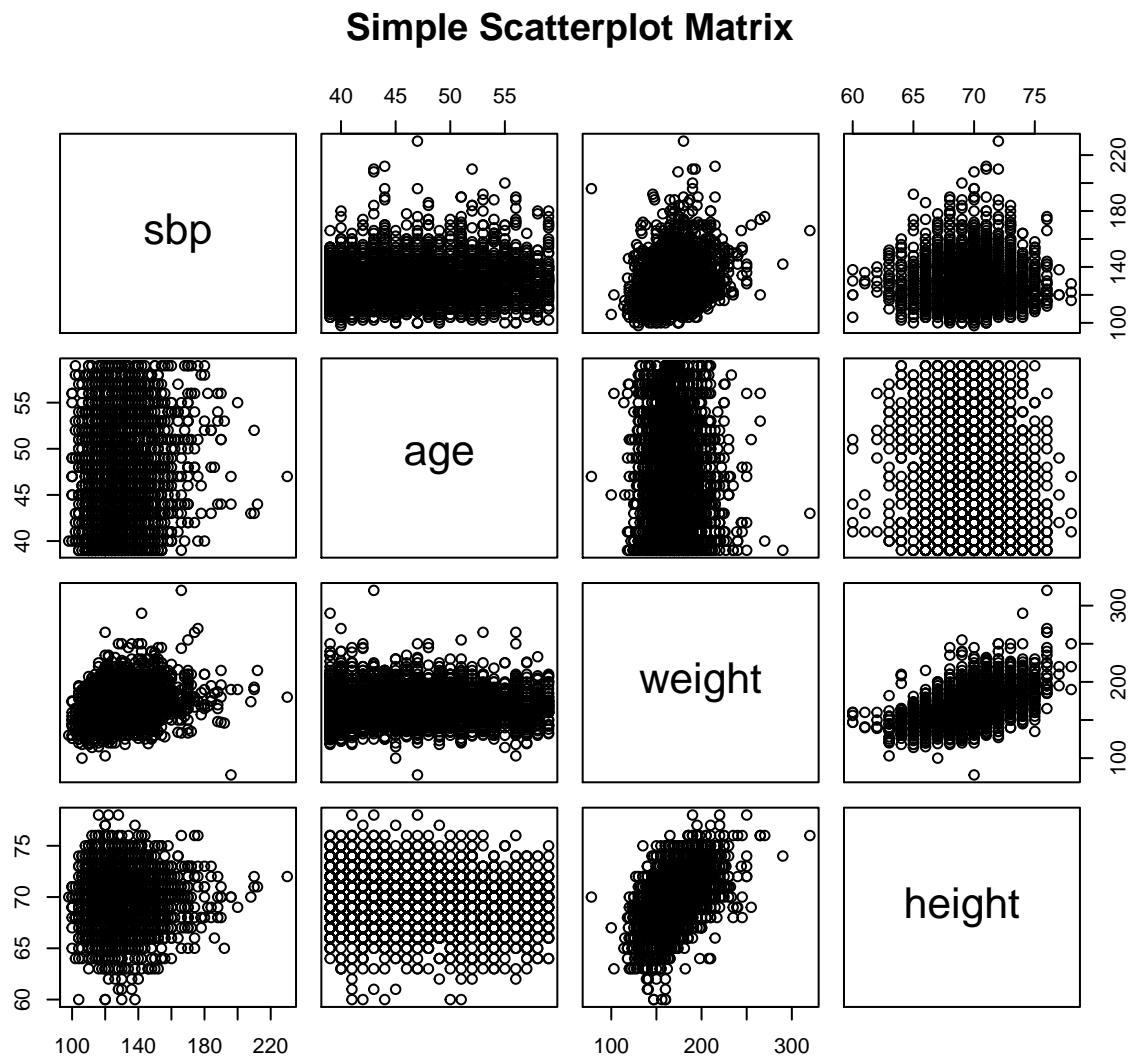
```
theme(strip.text = element_text(face="bold", size=rel(1.25), color="white"),
      strip.background = element_rect(fill="royalblue")) +
  labs(title = "Scatterplots of SBP vs. Weight within Behavior Pattern")
```



### 13.13 Scatterplot and Correlation Matrices

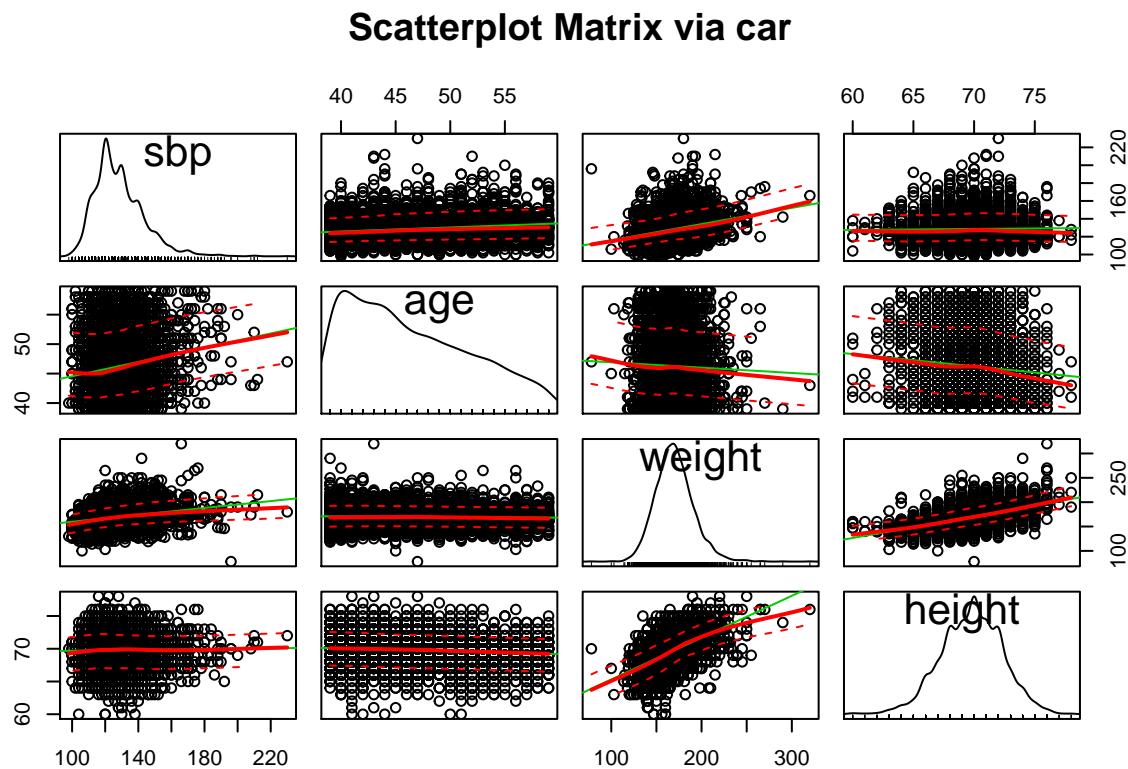
A **scatterplot matrix** can be very helpful in understanding relationships between multiple variables simultaneously. There are several ways to build such a thing, including the `pairs` function...

```
pairs (~ sbp + age + weight + height, data=wcgs, main="Simple Scatterplot Matrix")
```



### 13.13.1 Using the car package

Or, we can use the `scatterplotMatrix` function from the `car` package, which adds some detail and fitting to the plots, and places density estimates (with rug plots) on the diagonals.



### 13.13.2 Displaying a Correlation Matrix

```
wcgs %>%
  dplyr::select(sbp, age, weight, height) %>%
  cor() %>% # obtain correlation coefficients for this subgroup
  signif(., 3) # round them off to three significant figures before printing
```

	sbp	age	weight	height
sbp	1.0000	0.1660	0.2530	0.0184
age	0.1660	1.0000	-0.0344	-0.0954
weight	0.2530	-0.0344	1.0000	0.5330
height	0.0184	-0.0954	0.5330	1.0000

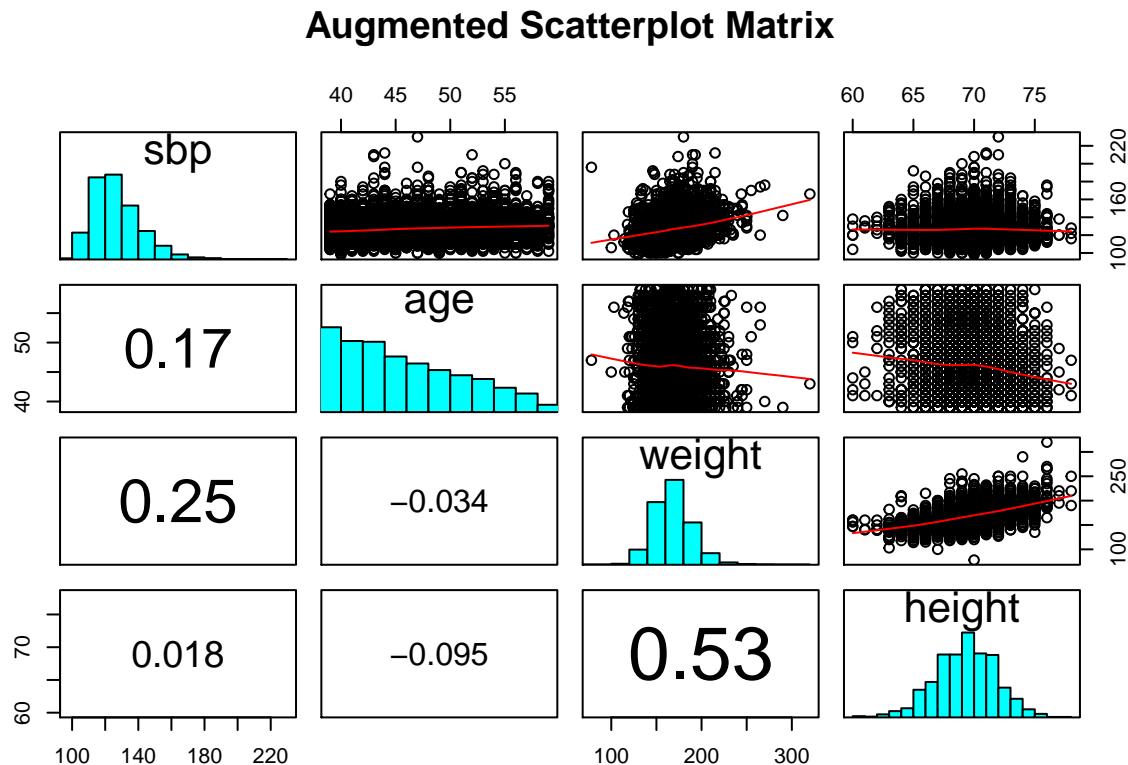
### 13.13.3 Augmented Scatterplot Matrix

Dr. Love's favorite way to augment a scatterplot matrix adds LOWESS smoothed lines in the upper panel, and correlations in the lower panel, with histograms down the diagonal. To do this, I revised two functions in the Love-boost script (these modifications come from Chang's R Graphics Cookbook), called `panel.hist` and `panel.cor`.

```
# requires Love-boost.R is sourced

pairs (~ sbp + age + weight + height, data=wcgs,
       main="Augmented Scatterplot Matrix",
```

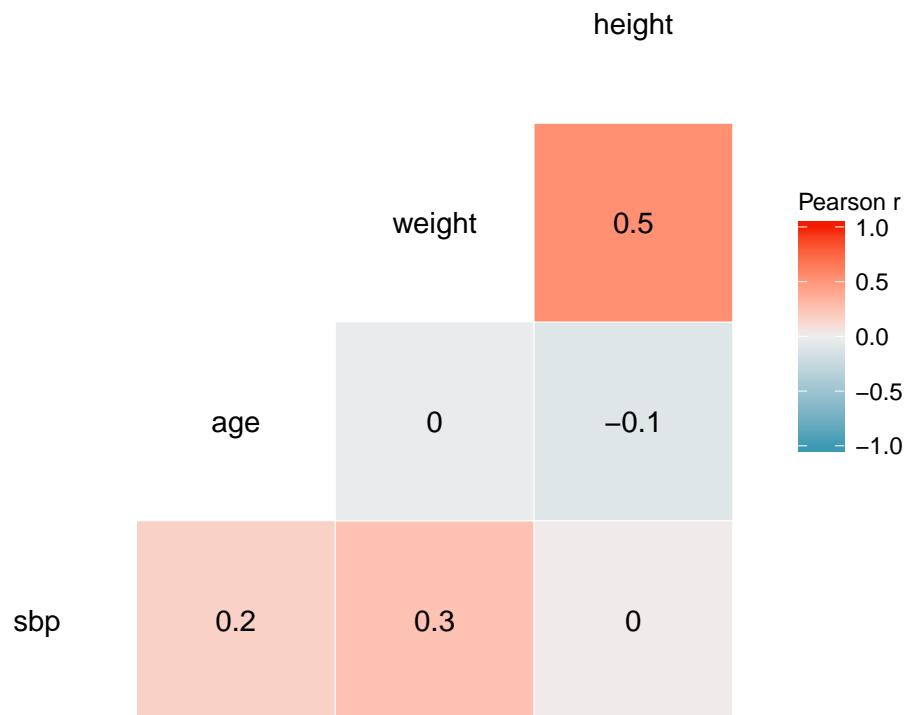
```
upper.panel = panel.smooth,
diag.panel = panel.hist,
lower.panel = panel.cor)
```



#### 13.13.4 Using the GGally package

The `ggplot2` system doesn't have a built-in scatterplot system. There are some nice add-ins in the world, though. One option I sort of like is in the `GGally` package, which can produce both correlation matrices and scatterplot matrices.

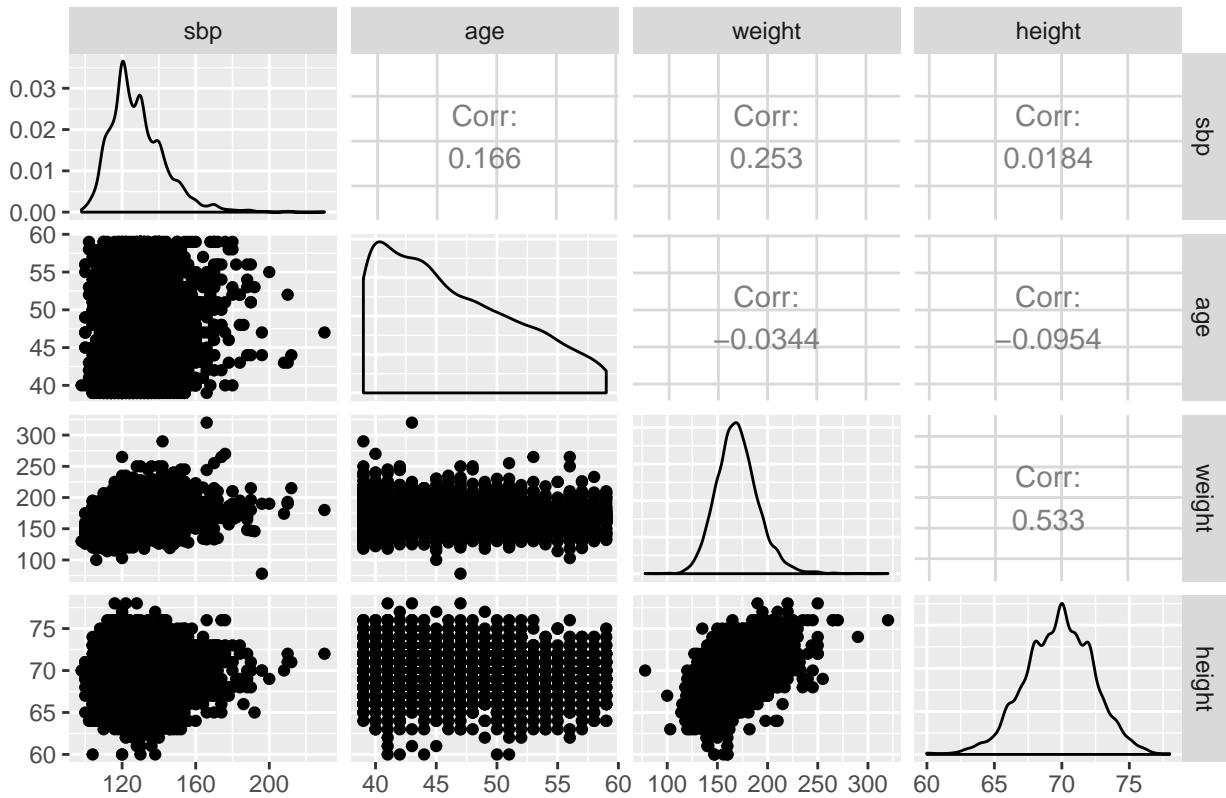
```
GGally::ggcorr(select(wcgs, sbp, age, weight, height),
               name = "Pearson r", label = TRUE)
```



The `ggpairs` function provides a density plot on each diagonal, Pearson correlations on the upper right and scatterplots on the lower left of the matrix.

```
GGally::ggpairs(select(wcgs, sbp, age, weight, height),  
                 title = "Scatterplot Matrix via ggpairs")
```

### Scatterplot Matrix via ggpairs



# Chapter 14

## Part A: A Few of the Key Points

### 14.1 Key Graphical Descriptive Summaries for Quantitative Data

- **Histograms** and their variants, including smooth density curves, and normal density functions based on the sample mean and sample standard deviation
- **Boxplots** and the like, including ridgeline plots and violin plots, that show more of the distribution in a compact format that is especially useful for comparisons
- **Normal QQ Plots** which are plots of the ordered data (technically, the order statistics) against certain quantiles of the Normal distribution - show curves to indicate skew, and “S” shaped arcs to indicate seriously heavy- or light-tailed distributions compared to the Normal.

### 14.2 Key Numerical Descriptive Summaries for Quantitative Data

- Measures of *Location* (including Central Tendency), such as the **mean**, **median**, **quantiles** and even the **mode**.
- Measures of *Spread*, including the **range**, **IQR** (which measures the variability of points near the center of the distribution), **standard deviation** (which is less appropriate as a summary measure if the data show substantial skew or heavy-tailedness), **variance**, **standard error**, **median absolute deviation** (which is less affected by outlying values in the tails of the distribution than a standard deviation).
- I'll mention the **coefficient of variation** (ratio of the standard deviation to the mean, expressed as a percentage, note that this is only appropriate for variables that take only positive values.)
- One Key Measure of *Shape* is nonparametric skew ( $\text{skew}_1$ ), which can be used to help confirm plot-based decisions about data shape.

### 14.3 The Empirical Rule - Interpreting a Standard Deviation

If the data are approximately Normally distributed, then the mean and median will be very similar, and there will be minimal skew and no large outlier problem.

Should this be the case, the mean and standard deviation describe the distribution well, and the **Empirical Rule** will hold reasonably well.

If the data are (approximately) Normally distributed, then

- About 68% of the data will fall within one standard deviation of the mean
- Approximately 95% of the data will fall within two standard deviations of the mean

- Approximately 99.7% of the data will fall within three standard deviations of the mean.

## 14.4 Identifying “Outliers” Using Fences and/or Z Scores

- Distributions can be symmetric, but still not Normally distributed, if they are either outlier-prone (heavy-tailed) or light-tailed.
- Outliers can have an important impact on other descriptive measures.
- John Tukey described **fences** which separated non-outlier from outlier values in a distribution. Generally, the fences are set 1.5 IQR away from the 25th and 75th percentiles in a boxplot.
- Or, we can use **Z scores** to highlight the relationship between values and what we might expect if the data were normally distributed.
- The Z score for an individual value is that value minus the data’s mean, all divided by the data’s standard deviation.
- If the data are normally distributed, we’d expect all but 5% of its observations to have Z scores between -2 and +2, for example.

## 14.5 Summarizing Bivariate Associations: Scatterplots and Regression Lines

- The most important tools are various **scatterplots**, often accompanied by **regression lines** estimated by the method of least squares, and by (loess) **smooths** which permit local polynomial functions to display curved relationships.
- In a multivariate setting, we will occasionally consider plots in the form of a **scatterplot matrix** to enable simultaneous comparisons of multiple two-way associations.
- We fit linear models to our data using the `lm` function, and we evaluate the models in terms of their ability to predict an outcome given a predictor, and through  $R^2$ , which is interpreted as the proportion of variation in the outcome accounted for by the model.

## 14.6 Summarizing Bivariate Associations With Correlations

- **Correlation coefficients**, of which by far the most commonly used is the **Pearson correlation**, which is a unitless (scale-free) measure of bivariate linear association for the variables X and Y, symbolized by  $r$ , and ranging from -1 to +1. The Pearson correlation is a function of the slope of the least squares regression line, divided by the product of the standard deviations of X and Y.
- Also relevant to us is the **Spearman rank correlation coefficient**, which is obtained by using the usual formula for a Pearson correlation, but on the ranks (1 = minimum,  $n$  = maximum, with average ranks are applied to the ties) of the X and Y values. This approach (running a correlation of the orderings of the data) substantially reduces the effect of outliers. The result still ranges from -1 to +1, with 0 indicating no monotone association.

## **Part B. Making Comparisons**



# Chapter 15

## Introduction to Part B

### 15.1 Point Estimation and Confidence Intervals

The basic theory of estimation can be used to indicate the probable accuracy and potential for bias in estimating based on limited samples. A point estimate provides a single best guess as to the value of a population or process parameter.

A confidence interval is a particularly useful way to convey to people just how much error one must allow for in a given estimate. In particular, a confidence interval allows us to quantify just how close we expect, for instance, the sample mean to be to the population or process mean. The computer will do the calculations; we need to interpret the results.

The key tradeoffs are cost vs. precision, and precision vs. confidence in the correctness of the statement. Often, if we are dissatisfied with the width of the confidence interval and want to make it smaller, we have little choice but to reconsider the sample – larger samples produce shorter intervals.

### 15.2 One-Sample Confidence Intervals and Hypothesis Testing

Very often, sample data indicate that something has happened – a change in the proportion, a shift in the mean, etc. Before we get excited, it's worth checking whether the apparent result might possibly be the result of random sampling error. The next few classes will be devoted to ideas of testing–seeing whether an apparent result might possibly be attributable to sheer randomness. Confidence intervals provide a way to assess this chance.

Statistics provides a number of tools for reaching an informed choice (informed by sample information, of course.) Which tool, or statistical method, to use depends on various aspects of the problem at hand. In addition, a  $p$  value, (often part of a computer output) gives an index of how much evidence we have that an apparent result is more than random.

### 15.3 Comparing Two Groups

In making a choice between two alternatives, questions such as the following become paramount.

- Is there a status quo?
- Is there a standard approach?
- What are the costs of incorrect decisions?
- Are such costs balanced?

The process of comparing the means/medians/proportions/rates of the populations represented by two independently obtained samples can be challenging, and such an approach is not always the best choice. Often, specially designed experiments can be more informative at lower cost (i.e. smaller sample size). As one might expect, using these more sophisticated procedures introduces trade-offs, but the costs are typically small relative to the gain in information.

When faced with such a comparison of two alternatives, a test based on **paired** data is often much better than a test based on two distinct, independent samples. Why? If we have done our experiment properly, the pairing lets us eliminate background variation that otherwise hides meaningful differences.

### 15.3.1 Model-Based Comparisons and ANOVA/Regression

Comparisons based on independent samples of quantitative variables are also frequently accomplished through other equivalent methods, including the analysis of variance approach and dummy variable regression, both of which produce the identical  $p$  values and confidence intervals to the pooled variance t test for the same comparison.

We will also discuss some of the main ideas in developing, designing and analyzing statistical experiments, specifically in terms of making comparisons. The ideas we will present in this section allow for the comparison of more than two populations in terms of their population means. The statistical techniques employed analyze the sample variance in order to test and estimate the population means and for this reason the method is called the analysis of variance (ANOVA), and we will discuss this approach alone, and within the context of a linear regression model using dummy or indicator variables.

## 15.4 Special Tools for Categorical Data

We will also turn briefly to some methods for dealing with qualitative, categorical variables. In particular, we begin with a test of how well the frequencies of various categories fit a theoretical set of probabilities. We also consider a test for the relation between two qualitative variables. We'll examine some of the key measures used in describing such relationships, like odds ratios and relative risks.

## 15.5 Our First Three Studies

We'll focus, for a while, on three studies, and the next three Sections of these Notes summarize each of them, graphically and numerically.

- The Serum Zinc study, which uses a single sample of quantitative data.
- The Lead in the Blood of Children study, which uses a *paired samples* design to compare two samples of quantitative data.
- A randomized controlled trial comparing ibuprofen vs. placebo in patients with sepsis, which uses an *independent samples* design to compare two samples of quantitative data.

## 15.6 Data Sets used in Part B

```
serzinc <- read_csv("data/serzinc.csv")
bloodlead <- read_csv("data/bloodlead.csv")
sepsis <- read_csv("data/sepsis.csv")
battery <- read.csv("data/battery.csv") %>%tbl_df
breakfast <- read.csv("data/breakfast.csv") %>%tbl_df
```

```
nyfs2 <- read.csv("data/nyfs2.csv") %>% tbl_df  
survey1 <- read.csv("data/surveyday1.csv") %>% tbl_df  
active2x3 <- read.csv("data/active2x3.csv") # deliberately NOT a tibble  
darwin <- read.csv("data/darwin.csv") %>% tbl_df
```

We'll also continue to make use of the `Love-boost.R` script of functions loaded in Section 2.



# Chapter 16

## The Serum Zinc Study

### 16.1 Serum Zinc Levels in 462 Teenage Males (`serzinc`)

The `serzinc` data include serum zinc levels in micrograms per deciliter that have been gathered for a sample of 462 males aged 15-17. My source for these data is Appendix B1 of Pagano and Gauvreau (2000). Serum zinc deficiency has been associated with anemia, loss of strength and endurance, and it is thought that 25% of the world's population is at risk of zinc deficiency. Such a deficiency can indicate poor nutrition, and can affect growth and vision, for instance. "Typical" values<sup>1</sup> are said to be 0.66-1.10 mcg/ml, which is 66 - 110 micrograms per deciliter.

```
serzinc
```

```
# A tibble: 462 x 2
  ID    zinc
  <chr> <int>
1 M-001   142
2 M-002    88
3 M-003    83
4 M-004   100
5 M-005   123
6 M-006    63
7 M-007   102
8 M-008    80
9 M-009   117
10 M-010   86
# ... with 452 more rows
```

### 16.2 Our Goal: A Confidence Interval for the Population Mean

After we assess the data a bit, and are satisfied that we understand it, our first inferential goal will be to produce a **confidence interval for the true (population) mean** of males age 15-17 based on this sample, assuming that these 462 males are a random sample from the population of interest, that each serum zinc level is drawn independently from an identical distribution describing that population.

To do this, we will have several different procedures available, including:

---

<sup>1</sup>Reference values for those over the age of 10 years at <http://www.mayomedicallaboratories.com/test-catalog/Clinical+and+Interpretive/8620>, visited 2017-08-17.

1. A confidence interval for the population mean based on a t distribution, when we assume that the data are drawn from an approximately Normal distribution, using the sample standard deviation. (Interval corresponding to a t test, and it will be a good choice when the data really are approximately Normally distributed.)
2. A resampling approach to generate a bootstrap confidence interval for the population mean, which does not require that we assume either that the population standard deviation is known, nor that the data are drawn from an approximately Normal distribution, but which has some other weaknesses.
3. A rank-based procedure called the Wilcoxon signed rank test can also be used to yield a confidence interval statement about the population pseudo-median, a measure of the population distribution's center (but not the population's mean).

## 16.3 Exploratory Data Analysis for Serum Zinc

### 16.3.1 Comparison to “Normal” Zinc Levels

Recall that the “Normal” zinc level would be between 66 and 110. What percentage of the sampled 462 teenagers meet that standard?

```
serzinc %>%
  count(zinc > 65 & zinc < 111) %>%
  mutate(proportion = n / sum(n), percentage = 100 * n / sum(n))
```

```
# A tibble: 2 x 4
`zinc > 65 & zinc < 111`   n proportion percentage
<lgl> <int>      <dbl>      <dbl>
1 FALSE     67      0.145     14.5
2 TRUE     395      0.855     85.5
```

### 16.3.2 Graphical Summaries

The code presented below builds:

- a histogram (with Normal model superimposed),
- a boxplot (with median notch) and
- a Normal Q-Q plot (with guiding straight line through the quartiles)

for the `zinc` results from the `serzinc` tibble. It does this while making use of several functions contained in the script `Love-boost.R`.

These functions include:

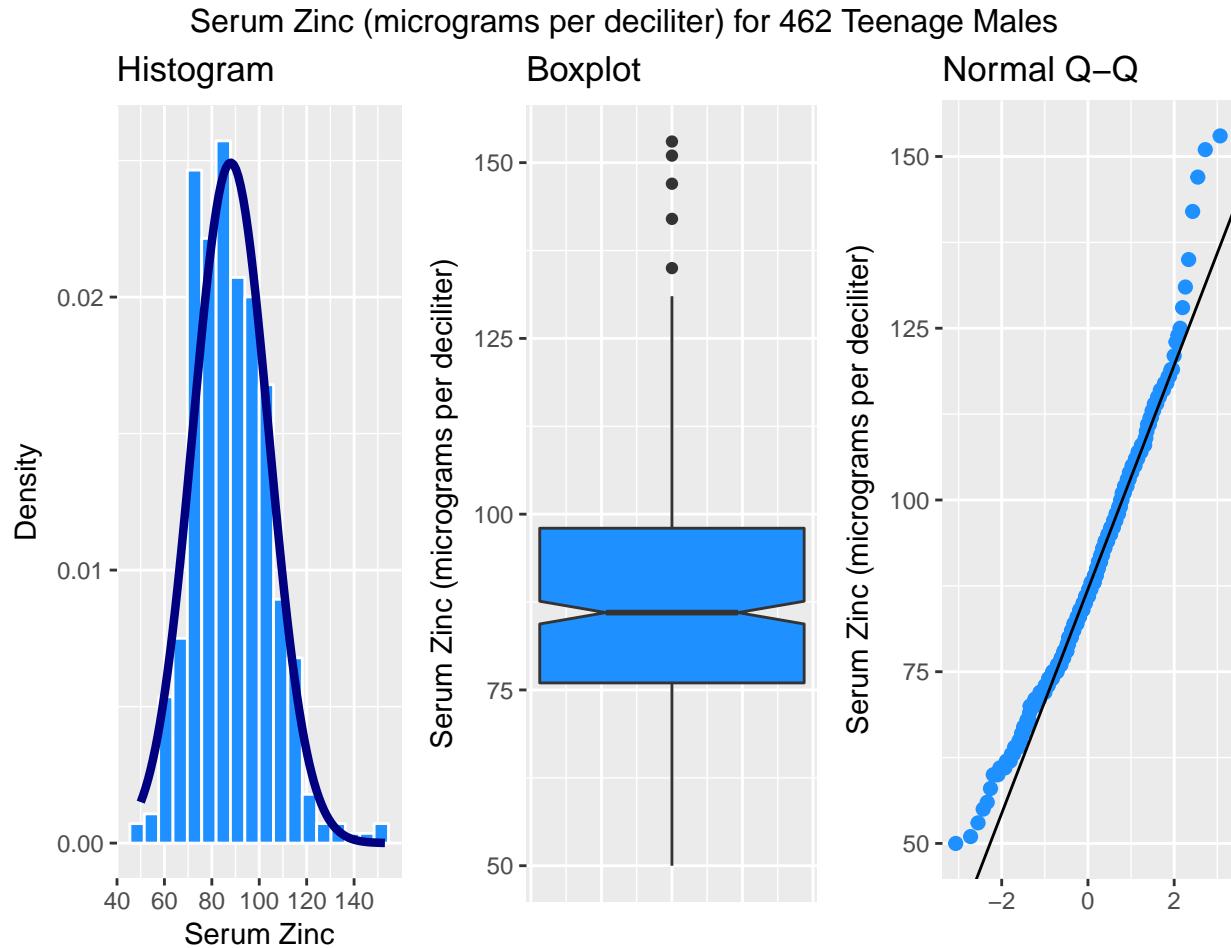
- `fd_bins` to estimate the Freedman-Diaconis bins setting for the histogram
- `qq_int` and `qq_slope` to facilitate the drawing of a line on the Normal Q-Q plot

```
p1 <- ggplot(serzinc, aes(x = zinc)) +
  geom_histogram(aes(y = ..density..), bins = fd_bins(serzinc$zinc),
                 fill = "dodgerblue", col = "white") +
  stat_function(fun = dnorm,
                args = list(mean = mean(serzinc$zinc),
                            sd = sd(serzinc$zinc)),
                lwd = 1.5, col = "navy") +
  labs(title = "Histogram",
       x = "Serum Zinc", y = "Density")
```

```
p2 <- ggplot(serzinc, aes(x = 1, y = zinc)) +
  geom_boxplot(fill = "dodgerblue", notch = TRUE) +
  theme(axis.text.x = element_blank(), axis.ticks.x = element_blank()) +
  labs(title = "Boxplot",
       y = "Serum Zinc (micrograms per deciliter)", x = "")

p3 <- ggplot(serzinc, aes(sample = zinc)) +
  geom_qq(col = "dodgerblue", size = 2) +
  geom_abline(intercept = qq_int(serzinc$zinc),
              slope = qq_slope(serzinc$zinc)) +
  labs(title = "Normal Q-Q",
       y = "Serum Zinc (micrograms per deciliter)", x = "")

gridExtra::grid.arrange(p1, p2, p3, nrow=1,
                       top = "Serum Zinc (micrograms per deciliter) for 462 Teenage Males")
```



These results include some of the more useful plots and numerical summaries when assessing shape, center and spread. The `zinc` data in the `serzinc` data frame appear to be slightly right skewed, with five outlier values on the high end of the scale, in particular.

You could potentially add `coord_flip()` + to the histogram, and this would have the advantage of getting all three plots oriented in the same direction, but then we (or at least I) lose the ability to tell the direction of skew at a glance from the direction of the histogram.

### 16.3.3 Numerical Summaries

This section describes some numerical summaries of interest to augment the plots in summarizing the center, spread and shape of the distribution of serum zinc among these 462 teenage males.

The tables below are built using two functions from the `Love-boost.R` script.

- `skew1` provides the `skew1` value for the `zinc` data and
- `Emp_Rule` provides the results of applying the 68-95-99.7 Empirical Rule to the `zinc` data.

```
pander(mosaic::favstats(serzinc$zinc))
```

min	Q1	median	Q3	max	mean	sd	n	missing
50	76	86	98	153	87.94	16	462	0

```
signif(skew1(serzinc$zinc),3)
```

```
[1] 0.121
```

The `skew1` value backs up our graphical assessment, that the data are slightly right skewed.

We can also assess how well the 68-95-99.7 Empirical Rule for a Normal distribution holds up for these data. Not too badly, as it turns out.

```
Emp_Rule(serzinc$zinc)
```

	count	proportion
Mean +/- 1 SD	323	0.6991
Mean +/- 2 SD	447	0.9675
Mean +/- 3 SD	458	0.9913
Entire Data Set	462	1

```
pander(psych::describe(serzinc$zinc))
```

Table 16.3: Table continues below

	vars	n	mean	sd	median	trimmed	mad	min	max
<b>X1</b>	1	462	87.94	16	86	87.17	16.31	50	153

	range	skew	kurtosis	se
<b>X1</b>	103	0.6191	0.8732	0.7446

Rounded to two decimal places, the standard deviation of the serum zinc data turns out to be 16, and so the standard error of the mean, shown as `se` in the `psych::describe` output, is 16 divided by the square root of the sample size,  $n = 462$ . This standard error is about to become quite important to us in building statistical inferences about the mean of the entire population of teenage males based on this sample.

# Chapter 17

## A Paired Sample Study: Lead in the Blood of Children

One of the best ways to eliminate a source of variation and the errors of interpretation associated with it is through the use of matched pairs. Each subject in one group is matched as closely as possible by a subject in the other group. If a 45-year-old African-American male with hypertension is given a [treatment designed to lower their blood pressure], then we give a second, similarly built 45-year old African-American male with hypertension a placebo.

- Good (2005), section 5.2.4

### 17.1 The Lead in the Blood of Children Study

Morton et al. (1982) studied the absorption of lead into the blood of children. This was a matched-sample study, where the exposed group of interest contained 33 children of parents who worked in a battery manufacturing factory (where lead was used) in the state of Oklahoma. Specifically, each child with a lead-exposed parent was matched to another child of the same age, exposure to traffic, and living in the same neighborhood whose parents did not work in lead-related industries. So the complete study had 66 children, arranged in 33 matched pairs. The outcome of interest, gathered from a sample of whole blood from each of the children, was lead content, measured in mg/dl.

One motivation for doing this study is captured in the Abstract from Morton et al. (1982).

It has been repeatedly reported that children of employees in a lead-related industry are at increased risk of lead absorption because of the high levels of lead found in the household dust of these workers.

The data are available in several places, including Table 5 of Pruzek and Helmreich (2009), in the `BloodLead` data set within the `PairedData` package in R, but we also make them available in the `bloodlead.csv` file. A table of the first three pairs of observations (blood lead levels for one child exposed to lead and the matched control) is shown below.

```
head(bloodlead, 3)
```

```
# A tibble: 3 x 3
  pair exposed control
  <chr>   <int>    <int>
1 P01      38       16
2 P02      23       18
```

3 P03 41 18

- In each pair, one child was exposed (to having a parent working in the factory) and the other was not.
- Otherwise, though, each child was very similar to its matched partner.
- The data under **exposed** and **control** are the blood lead content, in mg/dl.

Our primary goal will be to estimate the difference in lead content between the exposed and control children, and then use that sample estimate to make inferences about the difference in lead content between the population of all children like those in the exposed group and the population of all children like those in the control group.

### 17.1.1 Our Key Questions for a Paired Samples Comparison

1. What is the **population** under study?
  - All pairs of children living in Oklahoma near the factory in question, in which one had a parent working in a factory that exposed them to lead, and the other did not.
2. What is the **sample**? Is it representative of the population?
  - The sample consists of 33 pairs of one exposed and one control child.
  - This is a case-control study, where the children were carefully enrolled to meet the design criteria. Absent any other information, we're likely to assume that there is no serious bias associated with these pairs, and that assuming they represent the population effectively (and perhaps the broader population of kids whose parents work in lead-based industries more generally) may well be at least as reasonable as assuming they don't.
3. Who are the subjects / **individuals** within the sample?
  - Each of our 33 pairs of children includes one exposed child and one unexposed (control) child.
4. What **data** are available on each individual?
  - The blood lead content, as measured in mg/dl of whole blood.

### 17.1.2 Lead Study Caveats

Note that the children were not randomly selected from general populations of kids whose parents did and did not work in lead-based industries.

- To make inferences to those populations, we must make **strong assumptions** to believe, for instance, that the sample of exposed children is as representative as a random sample of children with similar exposures across the world would be.
- The researchers did have a detailed theory about how the exposed children might be at increased risk of lead absorption, and in fact as part of the study gathered additional information about whether a possible explanation might be related to the quality of hygiene of the parents (all of them were fathers, actually) who worked in the factory.
- This is an observational study, so that the estimation of a causal effect between parental work in a lead-based industry and children's blood lead content can be made, without substantial (and perhaps heroic) assumptions.

## 17.2 Exploratory Data Analysis for Paired Samples

We'll begin by adjusting the data in two ways.

- We'd like that first variable (`pair`) to be a `factor` rather than a `character` type in R, because we want to be able to summarize it more effectively. So we'll make that change.
- Also, we'd like to calculate the difference in lead content between the exposed and the control children in each pair, and we'll save that within-pair difference in a variable called `leaddir`. We'll take `leaddir` = `exposed - control` so that positive values indicate increased lead in the exposed child.

```
bloodlead <- bloodlead %>%
  mutate(pair = factor(pair),
        leaddir = exposed - control)

bloodlead
```

```
# A tibble: 33 x 4
  pair exposed control leaddir
  <fctr>   <int>    <int>    <int>
1 P01      38       16      22
2 P02      23       18       5
3 P03      41       18      23
4 P04      18       24     -6
5 P05      37       19      18
6 P06      36       11      25
7 P07      23       10      13
8 P08      62       15      47
9 P09      31       16      15
10 P10     34       18      16
# ... with 23 more rows
```

### 17.2.1 The Paired Differences

To begin, we focus on `leaddir` for our exploratory work, which is the `exposed - control` difference in lead content within each of the 33 pairs. So, we'll have 33 observations, as compared to the 462 in the serum zinc data, but most of the same tools are still helpful.

```
p1 <- ggplot(bloodlead, aes(x = leaddir)) +
  geom_histogram(aes(y = ..density..), bins = fd_bins(bloodlead$leaddir),
                 fill = "lightsteelblue4", col = "white") +
  stat_function(fun = dnorm,
                args = list(mean = mean(bloodlead$leaddir),
                            sd = sd(bloodlead$leaddir)),
                lwd = 1.5, col = "navy") +
  labs(title = "Histogram",
       x = "Diff. in Lead Content (mg/dl)", y = "Density") +
  theme_bw()

p2 <- ggplot(bloodlead, aes(x = 1, y = leaddir)) +
  geom_boxplot(fill = "lightsteelblue4", notch = TRUE) +
  theme(axis.text.x = element_blank(), axis.ticks.x = element_blank()) +
  labs(title = "Boxplot",
       y = "Difference in Blood Lead Content (mg/dl)", x = "") +
  theme_bw()

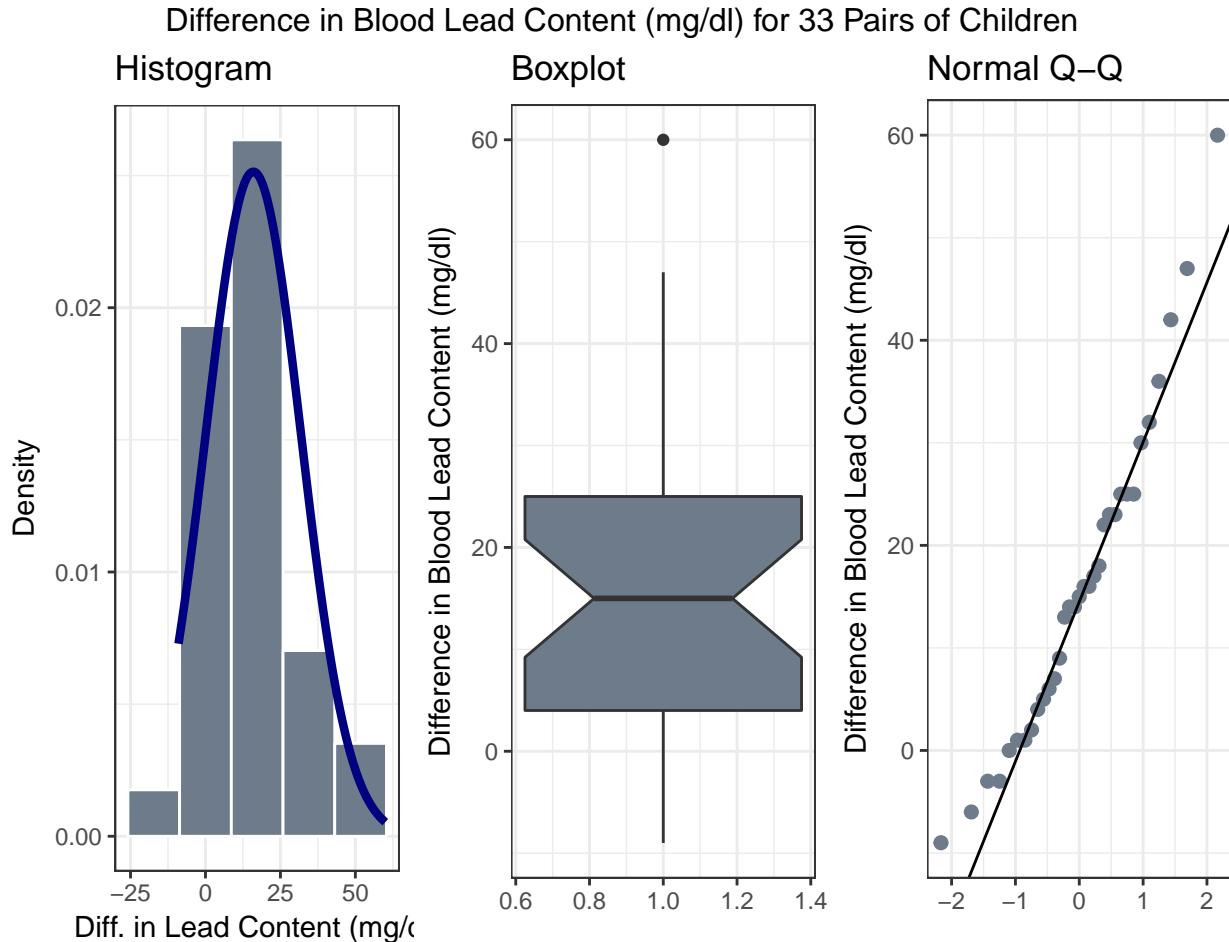
p3 <- ggplot(bloodlead, aes(sample = leaddir)) +
  geom_qq(col = "lightsteelblue4", size = 2) +
  geom_abline(intercept = qq_int(bloodlead$leaddir),
```

```

      slope = qq_slope(bloodlead$leaddir) +
labs(title = "Normal Q-Q",
y = "Difference in Blood Lead Content (mg/dl)", x = "") +
theme_bw()

gridExtra::grid.arrange(p1, p2, p3, nrow=1,
top = "Difference in Blood Lead Content (mg/dl) for 33 Pairs of Children")

```



Note that in all of this work, I plotted the paired differences. One obvious way to tell if you have paired samples is that you can pair every single subjects from one exposure group to the subjects in the other exposure group. Everyone has to be paired, so the sample sizes will always be the same in the two groups.

### 17.2.2 Numerical Summaries

```
pander(mosaic::favstats(bloodlead$leaddir))
```

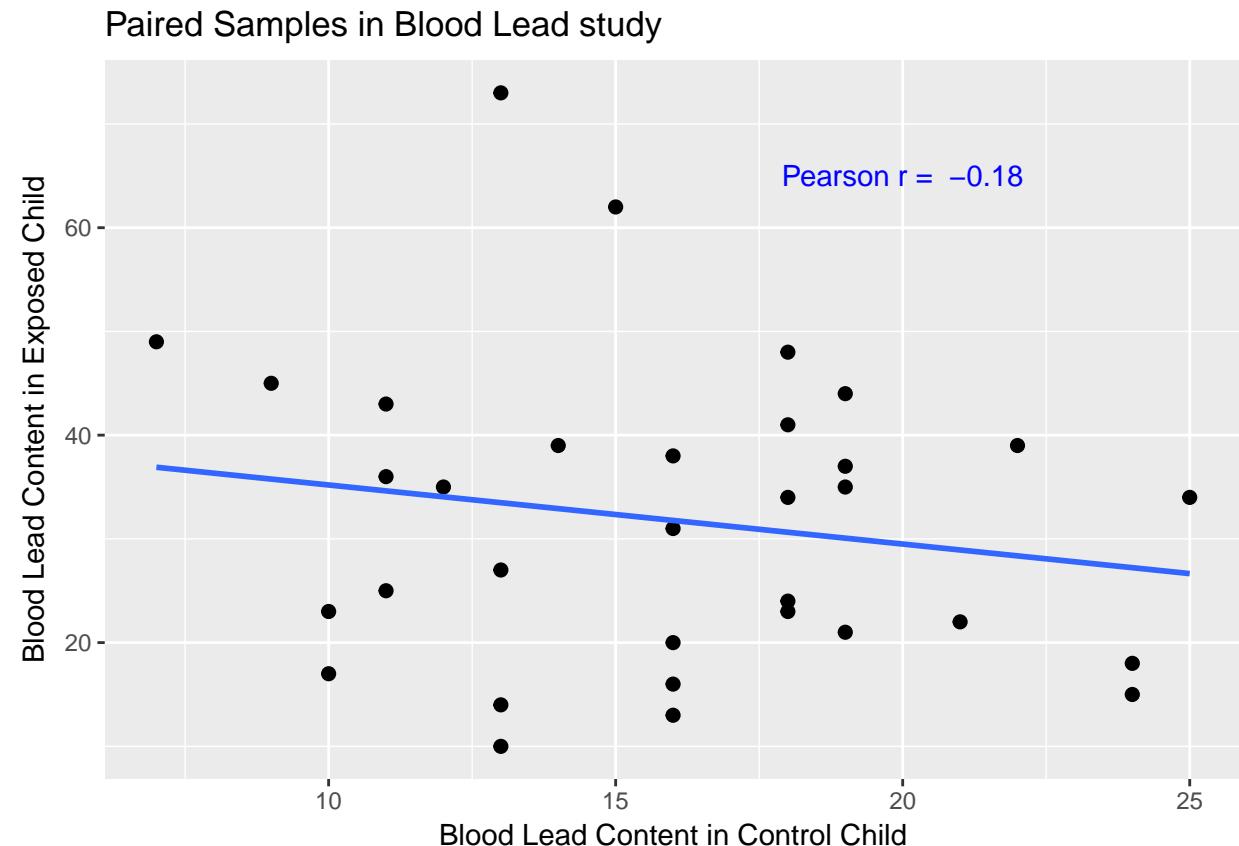
min	Q1	median	Q3	max	mean	sd	n	missing
-9	4	15	25	60	15.97	15.86	33	0

```
signif(skew1(bloodlead$leaddir), 3)
[1] 0.0611
```

### 17.2.3 Impact of Matching - Scatterplot and Correlation

Here, the data are paired by the study through matching on neighborhood, age and exposure to traffic. Each individual child's outcome value is part of a pair with the outcome value for his/her matching partner. We can see this pairing in several ways, perhaps by drawing a scatterplot of the pairs.

```
ggplot(bloodlead, aes(x = control, y = exposed)) +
  geom_point(size = 2) +
  geom_smooth(method = "lm", se = FALSE) +
  annotate("text", 20, 65, col = "blue",
           label = paste("Pearson r = ",
                         round(cor(bloodlead$control, bloodlead$exposed), 2))) +
  labs(title = "Paired Samples in Blood Lead study",
       x = "Blood Lead Content in Control Child",
       y = "Blood Lead Content in Exposed Child")
```



If there is a strong linear relationship (usually with a positive slope, thus positive correlation) between the paired outcomes, then the pairing will be more helpful in terms of improving statistical power of the estimates we build than if there is a weak relationship.

- The stronger the Pearson correlation coefficient, the more helpful pairing will be.

- Here, a straight line model using the control child's blood lead content accounts for about 3% of the variation in blood lead content in the exposed child.
- As it turns out, pairing will have only a modest impact here on the inferences we draw in the study.

### 17.3 Looking at the Individual Samples: Tidying the Data with `gather`

For the purpose of estimating the difference between the exposed and control children, the summaries of the paired differences are what we'll need.

In some settings, however, we might also look at a boxplot, or violin plot, or ridgeline plot that showed the distributions of exposed and control children separately. But we will run into trouble because one variable (blood lead content) is spread across multiple columns (control and exposed.) The solution is to `gather` up that variable so as to build a new, tidy tibble.

Because the data aren't *tidied* here, so that we have one row for each subject and one column for each variable, we have to do some work to get them in that form for our usual plotting strategy to work well. For more on this approach (gathering and its opposite, spreading the data), visit the Tidy data chapter in Grolemund and Wickham (2017).

```
blead_tidied <- bloodlead %>%
  gather(control, exposed, key = "status", value = "leadcontent") %>%
  mutate(status = factor(status)) %>%
  select(-leaddir)

blead_tidied
```

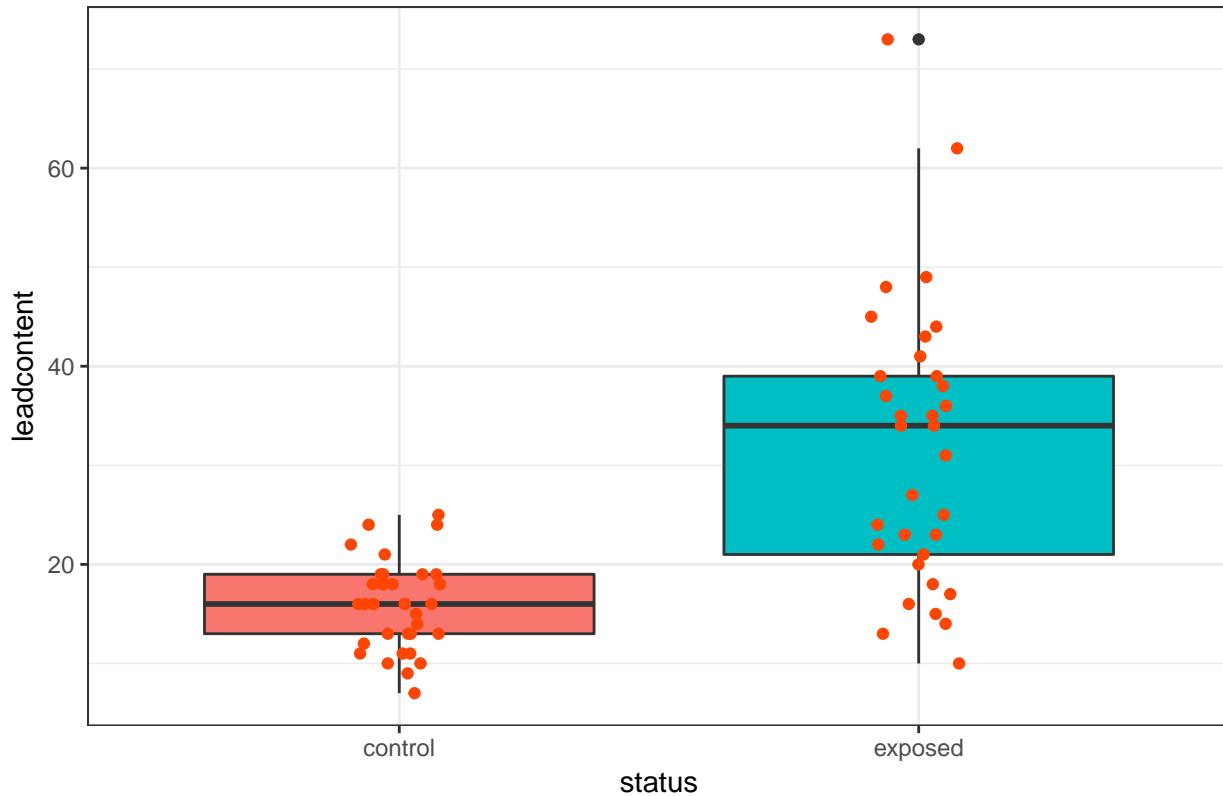
	pair	status	leadcontent
1	P01	control	16
2	P02	control	18
3	P03	control	18
4	P04	control	24
5	P05	control	19
6	P06	control	11
7	P07	control	10
8	P08	control	15
9	P09	control	16
10	P10	control	18
# ...			with 56 more rows

And now, we can plot as usual to compare the two samples.

First, we'll look at a boxplot, showing all of the data.

```
ggplot(blead_tidied, aes(x = status, y = leadcontent, fill = status)) +
  geom_boxplot() +
  geom_jitter(width = 0.1, height = 0, color = "orangered") +
  guides(fill = FALSE) +
  labs(title = "Boxplot of Lead Content in Exposed and Control kids") +
  theme_bw()
```

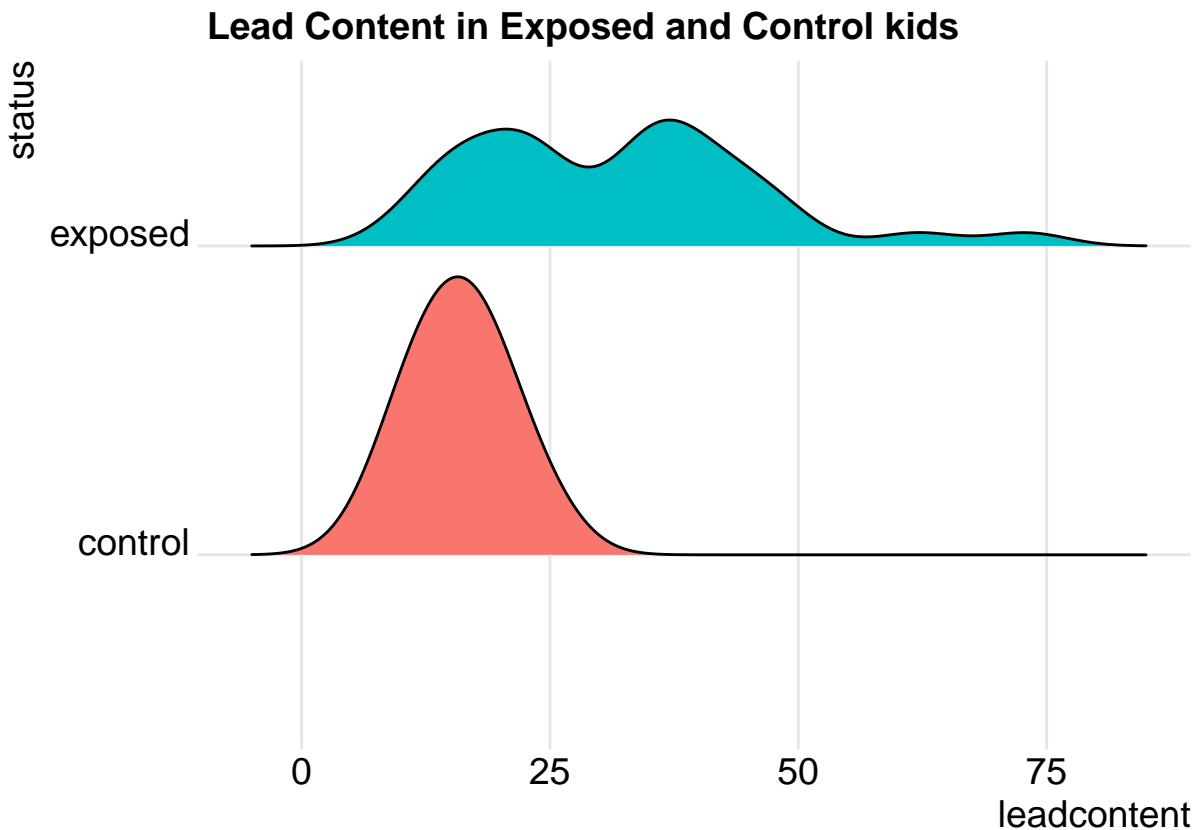
### Boxplot of Lead Content in Exposed and Control kids



We'll also look at a ridgeline plot, because Dr. Love likes them, even though they're really more useful when we're comparing more than two samples.

```
ggplot(blead_tidied, aes(x = leadcontent, y = status, fill = status)) +
  ggridges::geom_density_ridges(scale = 0.9) +
  guides(fill = FALSE) +
  labs(title = "Lead Content in Exposed and Control kids") +
  ggridges::theme_ridges()
```

Picking joint bandwidth of 4.01



Both the center and the spread of the distribution are substantially larger in the exposed group than in the matched controls. Of course, numerical summaries show these patterns, too.

```
blead_tidied %>% group_by(status) %>%
  summarise(n = n(),
            median = median(leadcontent),
            Q1 = quantile(leadcontent, 0.25),
            Q3 = quantile(leadcontent, 0.75),
            mean = mean(leadcontent),
            sd = sd(leadcontent))

# A tibble: 2 x 7
  status     n median     Q1     Q3   mean     sd
  <fctr> <int> <dbl> <dbl> <dbl> <dbl>
1 control    33     16     13     19  15.9  4.54
2 exposed    33     34     21     39  31.8 14.41
```

# Chapter 18

## A Study Comparing Two Independent Samples: Ibuprofen in Sepsis Trial

### 18.1 The Ibuprofen in Sepsis Randomized Clinical Trial

We will be working with a sample from the Ibuprofen in Sepsis study, as reported in Bernard et al. (1997). My source for these data is Dupont (2002).

Ibuprofen has been shown to have effects on sepsis in humans, but because of their small samples (fewer than 30 patients), previous studies have been inadequate to assess effects on mortality. We sought to determine whether ibuprofen can alter rates of organ failure and mortality in patients with the sepsis syndrome, how the drug affects the increased metabolic demand in sepsis (e.g., fever, tachypnea, tachycardia, hypoxemia, and lactic acidosis), and what potential adverse effects the drug has in the sepsis syndrome.

- Bernard et al. (1997), Abstract.

In this study, patients meeting specific criteria (including elevated temperature) for a diagnosis of sepsis were recruited if they fulfilled an additional set of study criteria (see Bernard et al. (1997)) in the intensive care unit at one of seven participating centers. The full trial involved 455 patients, of which our sample includes 300. 150 of our patients were randomly assigned to the Ibuprofen group and 150 to the Placebo group. In either case, the patient received intravenous treatment (ibuprofen or placebo.) This was also a *double-blind* study, where neither the patients nor their care providers know, during the execution of the trial, what intervention group was assigned to each patient.

For the moment, we will focus on two variables:

- **treat**, which specifies the treatment group (Ibuprofen or Placebo), which was assigned via randomization to each patient, and
- **temp\_drop**, the outcome of interest, measured as the change from baseline to 2 hours later in degrees Celsius. Positive values indicate improvement, that is, a *drop* in temperature over the 2 hours following the baseline measurement.

The data in the `sepsis.csv` file also contains the subject's

- *id*, which is just a code
- *race* (three levels: White, AfricanA or Other)
- *apache* = baseline APACHE II score, a severity of disease score ranging from 0 to 71 with higher scores indicating more severe disease and a higher mortality risk
- *temp\_0* = baseline temperature, degrees Celsius.

but we'll ignore those for now.

```
sepsis
```

```
# A tibble: 300 x 6
  id      treat    race apache temp_0 temp_drop
  <chr>   <chr>   <chr>  <int>   <dbl>     <dbl>
1 S002 Ibuprofen AfricanA    14   38.7      1.4
2 S004 Ibuprofen White       3    38.3      0.4
3 S005 Placebo   White       5    38.6      0.0
4 S006 Ibuprofen White      13   38.2     -0.2
5 S009 Ibuprofen White      25   38.2      0.6
6 S011 Ibuprofen White      21   38.1     -0.4
7 S012 Placebo   White      14   38.6     -0.1
8 S014 Placebo   White      23   37.9      0.3
9 S016 Placebo   White      16   38.1      0.1
10 S020 Ibuprofen Other      20   39.2      1.5
# ... with 290 more rows
```

```
sepsis <- sepsis %>%
  mutate(treat = factor(treat),
        race = factor(race))
```

```
summary(select(sepsis, treat, temp_drop))
```

	treat	temp_drop
Ibuprofen:150	Min.	: -2.700
Placebo :150	1st Qu.	: -0.100
	Median	: 0.300
	Mean	: 0.308
	3rd Qu.	: 0.700
	Max.	: 3.100

Again, the complete study included 455 patients, but our sample includes 300. We have exactly 150 in the Ibuprofen group and 150 in the Placebo group, as it turns out. I picked the sample so as to exclude patients with missing values for our outcome of interest, and then selected a random sample of 150 Ibuprofen and 150 Placebo patients from the rest of the group, and converted the temperatures and changes from Fahrenheit to Celsius.

### 18.1.1 Matched Pairs vs. Two Independent Samples

These data were obtained from two independent samples, rather than as matched pairs.

- Remember that if the sample sizes were different, we'd know we have independent samples, because matched pairs requires that each subject in the “treated” group be matched to a single, unique member of the “control” group, and thus that we have exactly as many “treated” as “control” subjects.
- But having as many subjects in one treatment group as the other (which is called a *balanced design*) is only necessary, and not sufficient, for us to conclude that matched pairs are used.
- We only have matched pairs if each individual observation in the “treatment” group is matched to one and only one observation in the “control” group by the way in which the data were gathered.
  - Paired data can arise in several ways. The most common is a “pre-post” study where subjects are measured both before and after an exposure happens. In observational studies, we often match up subjects who did and did not receive an exposure so as to account for differences on things like age, sex, race and other covariates. This, of course, is what happens in the Lead in the Blood of Children study from Section 17.

- If the data are from paired samples, we should (and in fact) must form paired differences, with no subject left unpaired.
- If we cannot line up the data comparing two samples of quantitative data so that the links between the individual “treated” and “control” observations to form matched pairs are evident, then the data are not paired.

As Bock, Velleman, and De Veaux (2004) suggest,

... if you know the data are paired, you can take advantage of that fact - in fact, you *must* take advantage of it. ... You must decide whether the data are paired from understanding how they were collected and what they mean. ... There is no test to determine whether the data are paired.

### 18.1.2 Our Key Questions for an Independent Samples Comparison

1. What is the **population** under study?
  - All patients in the intensive care unit with sepsis who meet the inclusion and exclusion criteria of the study, at the entire population of health centers like the ones included in the trial.
2. What is the **sample**? Is it representative of the population?
  - The sample consists of 300 patients. It is a convenient sample from the population under study.
  - This is a randomized clinical trial. 150 of the patients were assigned to Ibuprofen, and the rest to Placebo. It is this treatment assignment that is randomized, not the selection of the sample as a whole.
  - In expectation, randomization of individuals to treatments, as in this study, should be expected to eliminate treatment selection bias.
3. Who are the subjects / **individuals** within the sample?
  - 150 patients who received Ibuprofen and a completely different set of 150 patients who received Placebo.
  - There is no match or link between the patients. They are best thought of as independent samples.
4. What **data** are available on each individual?
  - The key variables are the treatment indicator (Ibuprofen or Placebo) and the outcome (drop in temperature in the 2 hours following administration of the randomly assigned treatment.)

### 18.1.3 RCT Caveats

The placebo-controlled, double-blind randomized clinical trial, especially if pre-registered, is often considered the best feasible study for assessing the effectiveness of a treatment. While that's not always true, it is a very solid design. The primary caveat is that the patients who are included in such trials are rarely excellent representations of the population of potentially affected patients as a whole.

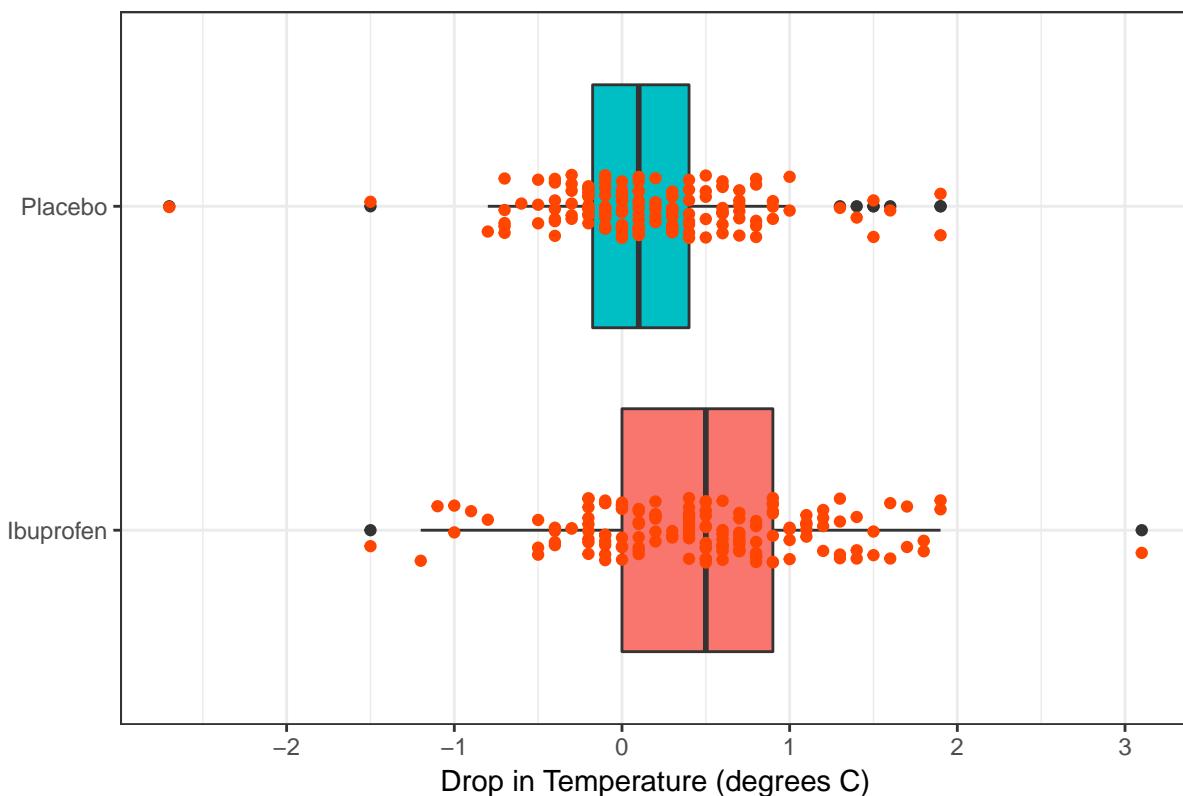
## 18.2 Exploratory Data Analysis

First, we'll look at a boxplot, showing all of the individual data as added-on dots.

```
ggplot(sepsis, aes(x = treat, y = temp_drop, fill = treat)) +
  geom_boxplot() +
  geom_jitter(width = 0.1, height = 0, color = "orangered") +
  guides(fill = FALSE) +
  labs(title = "Boxplot of Temperature Drop in Sepsis Patients",
       x = "", y = "Drop in Temperature (degrees C)") +
```

```
coord_flip() +
theme_bw()
```

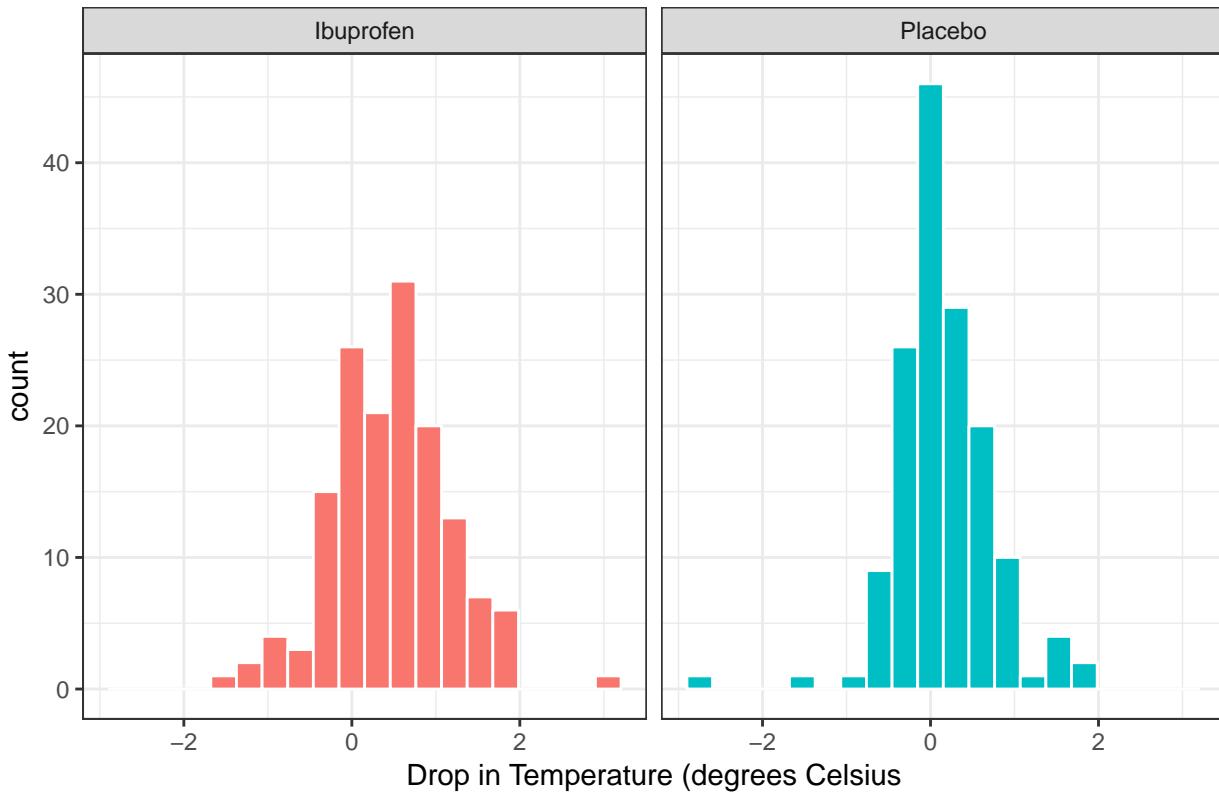
Boxplot of Temperature Drop in Sepsis Patients



Next, we'll consider faceted histograms of the data.

```
ggplot(sepsis, aes(x = temp_drop, fill = treat)) +
  geom_histogram(color = "white", bins = 20) +
  guides(fill = FALSE) +
  labs(title = "Histograms of Temperature Drop in Sepsis Patients",
       x = "Drop in Temperature (degrees Celsius)") +
  theme_bw() +
  facet_wrap(~ treat)
```

### Histograms of Temperature Drop in Sepsis Patients

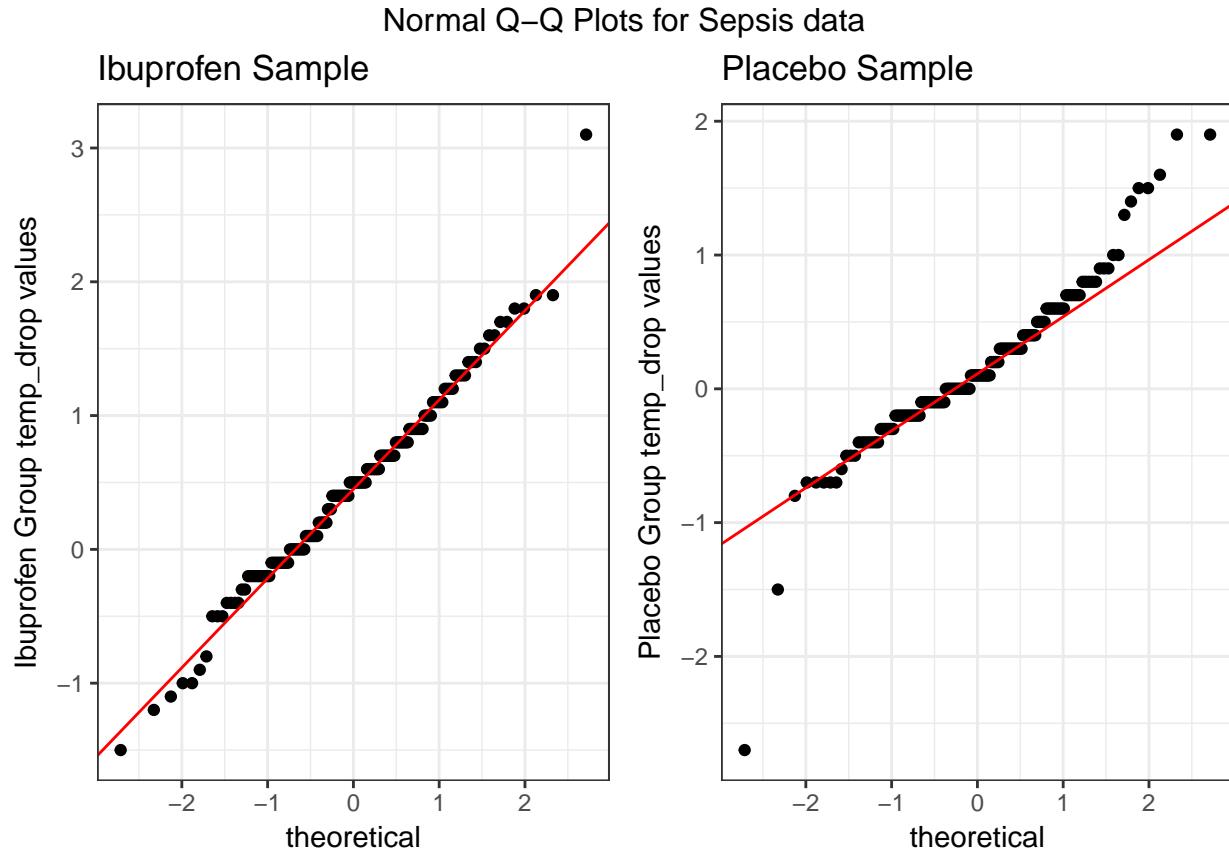


Here's a pair of Normal Q-Q plots. It's not hard to use a Normal model to approximate the Ibuprofen data, but such a model is probably not a good choice for the Placebo results.

```
p1 <- sepsis %>%
  filter(treat == "Ibuprofen") %>%
  ggplot(aes(sample = temp_drop)) +
  geom_qq() +
  geom_abline(intercept = qq_int(filter(sepsis, treat == "Ibuprofen")$temp_drop),
              slope = qq_slope(filter(sepsis, treat == "Ibuprofen")$temp_drop),
              col = "red") +
  labs(title = "Ibuprofen Sample", y = "Ibuprofen Group temp_drop values") +
  theme_bw()

p2 <- sepsis %>%
  filter(treat == "Placebo") %>%
  ggplot(aes(sample = temp_drop)) +
  geom_qq() +
  geom_abline(intercept = qq_int(filter(sepsis, treat == "Placebo")$temp_drop),
              slope = qq_slope(filter(sepsis, treat == "Placebo")$temp_drop),
              col = "red") +
  labs(title = "Placebo Sample", y = "Placebo Group temp_drop values") +
  theme_bw()

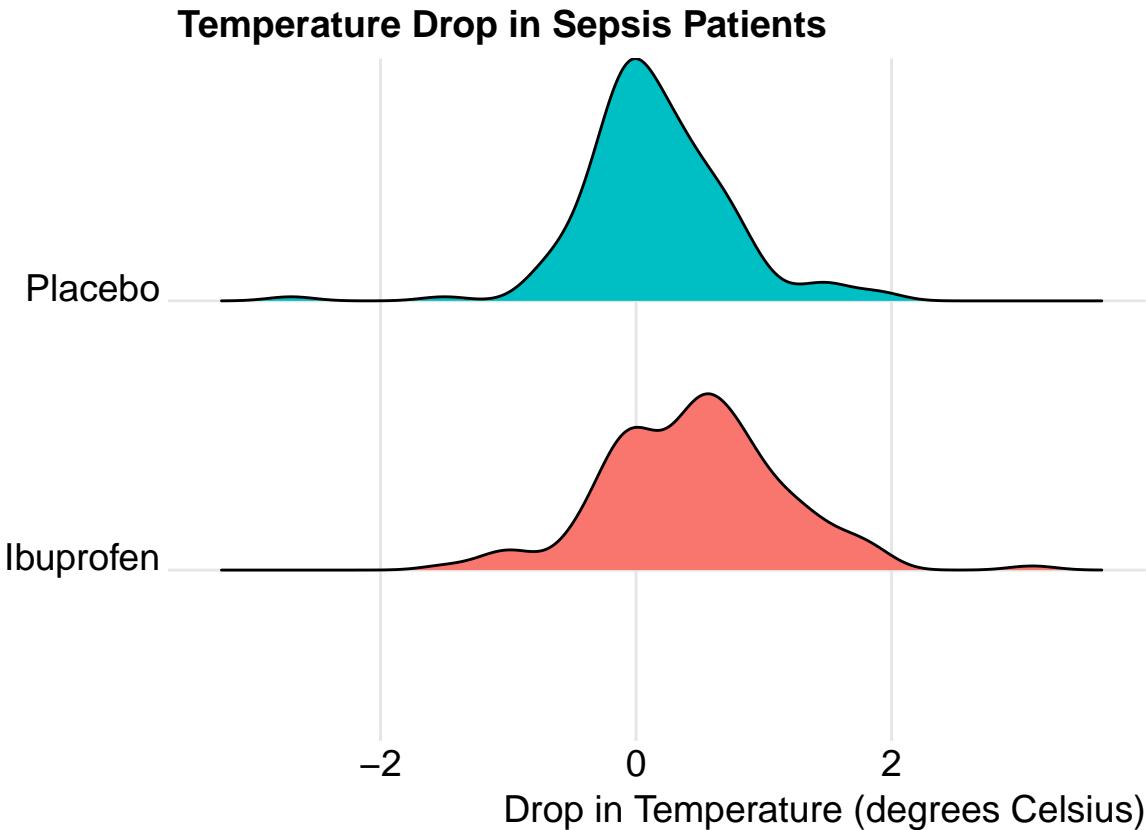
gridExtra::grid.arrange(p1, p2, nrow = 1, top = "Normal Q-Q Plots for Sepsis data")
```



We'll also look at a ridgeline plot.

```
ggplot(sepsis, aes(x = temp_drop, y = treat, fill = treat)) +
  ggridges::geom_density_ridges(scale = 0.9) +
  guides(fill = FALSE) +
  labs(title = "Temperature Drop in Sepsis Patients",
       x = "Drop in Temperature (degrees Celsius)", y = "") +
  ggridges::theme_ridges()
```

Picking joint bandwidth of 0.182



The center of the ibuprofen distribution is shifted a bit towards the more positive (greater improvement) direction, it seems, than is the distribution for the placebo patients. Here are some key numerical summaries, within the treatment groups, which buoy this conclusion.

```
sepsis %>% group_by(treat) %>%
  summarise(n = n(),
            median = median(temp_drop),
            Q1 = quantile(temp_drop, 0.25),
            Q3 = quantile(temp_drop, 0.75),
            mean = mean(temp_drop),
            sd = sd(temp_drop))

# A tibble: 2 x 7
  treat     n median     Q1     Q3   mean     sd
  <fctr> <int>  <dbl>  <dbl>  <dbl>  <dbl>  <dbl>
1 Ibuprofen 150     0.5  0.000    0.9  0.464  0.688
2 Placebo    150     0.1 -0.175    0.4  0.153  0.571
```



# Chapter 19

## Confidence Intervals for a Single Sample of Quantitative Data

Suppose that we are interested in learning something about a population or process, from which we can obtain a sample that consists of a subset of potential results from that population or process. The main goal for many of the parametric models that are a large part of statistics is to estimate population parameters, like a population mean, or regression coefficient, on the basis of a sample. When we do this, we want to describe not only our best guess at the parameter – referred to as a *point estimate*, but also say something useful about the uncertainty in our estimate, to let us more completely assess what the data have to tell us. A key tool for doing this is a **confidence interval**, described here in some detail.

Essentially every textbook on introductory statistics describes the development of a confidence interval, at least for a mean. Good supplemental resources include Diez, Barr, and Çetinyaka-Rundel (n.d.), Bock, Velleman, and De Veaux (2004) and Pagano and Gauvreau (2000), for instance.

We'll develop confidence intervals to compare parameters about two populations (either through matched pairs or independent samples) with confidence intervals soon. Here, we'll consider the problem of estimating a confidence interval to describe the mean (or median) of the population represented by a single sample of quantitative data. Our main example uses data from the Serum Zinc study, as described in Section 16.

### 19.1 Defining a Confidence Interval

A confidence interval for a population or process mean uses data from a sample (and perhaps some additional information) to identify a range of potential values for the population mean, which, if certain assumptions hold, can be assumed to provide a reasonable estimate for the true population mean. A confidence interval consists of:

1. An interval estimate describing the population parameter of interest (here the population mean), and
2. A probability statement, expressed in terms of a confidence level.

### 19.2 Estimating the Population Mean from the Serum Zinc data

As an example, suppose that we are willing to assume that the mean serum zinc level across the entire population of teenage males,  $\mu$ , follows a Normal distribution (and so, summarizing it with a mean is a rational thing to do.) Suppose that we are also willing to assume that the 462 teenage males contained in the `serzinc` tibble are a random sample from that complete population. While we know the mean of the sample of 462 boys, we don't know  $\mu$ , the mean across all teenage males. So we need to estimate it.

Later, we will find that, with these assumptions in place, we can find a 90% confidence interval for the mean serum zinc level across the entire population of teenage males. This 90% confidence interval for  $\mu$  turns out to be (86.71, 89.16) micrograms per deciliter. How would you interpret this result?

- Some people think this means that there is a 90% chance that the true mean of the population,  $\mu$ , falls between 86.71 and 89.16 micrograms per deciliter. That's not correct.
- The population mean is a constant **parameter** of the population of interest. That constant is not a random variable, and does not change. So the actual probability of the population mean falling inside that range is either 0 or 1.
- Our confidence is in our process.
  - It's in the sampling method (random sampling) used to generate the data, and in the assumption that the population follows a Normal distribution.
  - It's captured in our accounting for one particular type of error (called *sampling error*) in developing our interval estimate, while assuming all other potential sources of error are negligible.

So, what's closer to the truth is:

- If we used this same method to sample data from the true population of teenage males, and built 100 such 90% confidence intervals, then about 90 of them would contain the true population mean.

### 19.3 Confidence vs. Significance Level

We've estimated a 90% confidence interval for the population mean serum zinc level among teenage boys using the `serzinc` data.

- We call  $100(1-\alpha)\%$ , here, 90%, or 0.90, the *confidence* level, and
- $\alpha = 10\%$ , or 0.10 is called the *significance* level.

If we had instead built a series of 100 different 95% confidence intervals, then about 95 of them would contain the true value of  $\mu$ .

Let's look more closely at the issue of estimating a population **mean** based on a sample of observations. We will need three critical pieces - the sample, the confidence level, and the margin of error, which is based on the standard error of a sample mean, when we are estimating a population mean.

### 19.4 The Standard Error of a Sample Mean

The standard error, generally, is the name we give to the standard deviation associated with any particular parameter estimate.

- If we are using a sample mean based on a sample of size  $n$  to estimate a population mean, the **standard error of that sample mean** is  $\sigma/\sqrt{n}$ , where  $\sigma$  is the standard deviation of the measurements in the population.
- We often estimate this particular standard error with its sample analogue,  $s/\sqrt{n}$ , where  $s$  is the sample standard deviation.
- Other statistics have different standard errors.
  - $\sqrt{p(1-p)/n}$  is the standard error of the sample proportion  $p$  estimated using a sample of size  $n$ .
  - $\sqrt{\frac{1-r^2}{n-2}}$  is the standard error of the sample Pearson correlation  $r$  estimated using  $n$  pairs of observations.

In developing a confidence interval for a population mean, we may be willing to assume that the data in our sample are drawn from a Normally distributed population. If so, the most common and useful means of

building a confidence interval makes use of the t distribution (sometimes called Student's t) and the notion of a *standard error*.

## 19.5 The t distribution and Confidence Intervals for $\mu$

In practical settings, we will use the t distribution to estimate a confidence interval from a population mean whenever we:

- are willing to assume that the sample is drawn at random from a population or process with a Normal distribution,
- are using our sample to estimate both the mean and standard deviation, and
- have a small sample size.

### 19.5.1 The Formula

We can build a  $100(1-\alpha)\%$  confidence interval using the *t* distribution, using the sample mean  $\bar{x}$ , the sample size  $n$ , and the sample standard deviation  $s$ .

The two-sided  $100(1-\alpha)\%$  confidence interval (based on a *t* test) is:

$$\bar{x} \pm t_{\alpha/2, n-1}(s/\sqrt{n})$$

where  $t_{\alpha/2, n-1}$  is the value that cuts off the top  $\alpha/2$  percent of the *t* distribution, with  $n - 1$  degrees of freedom.

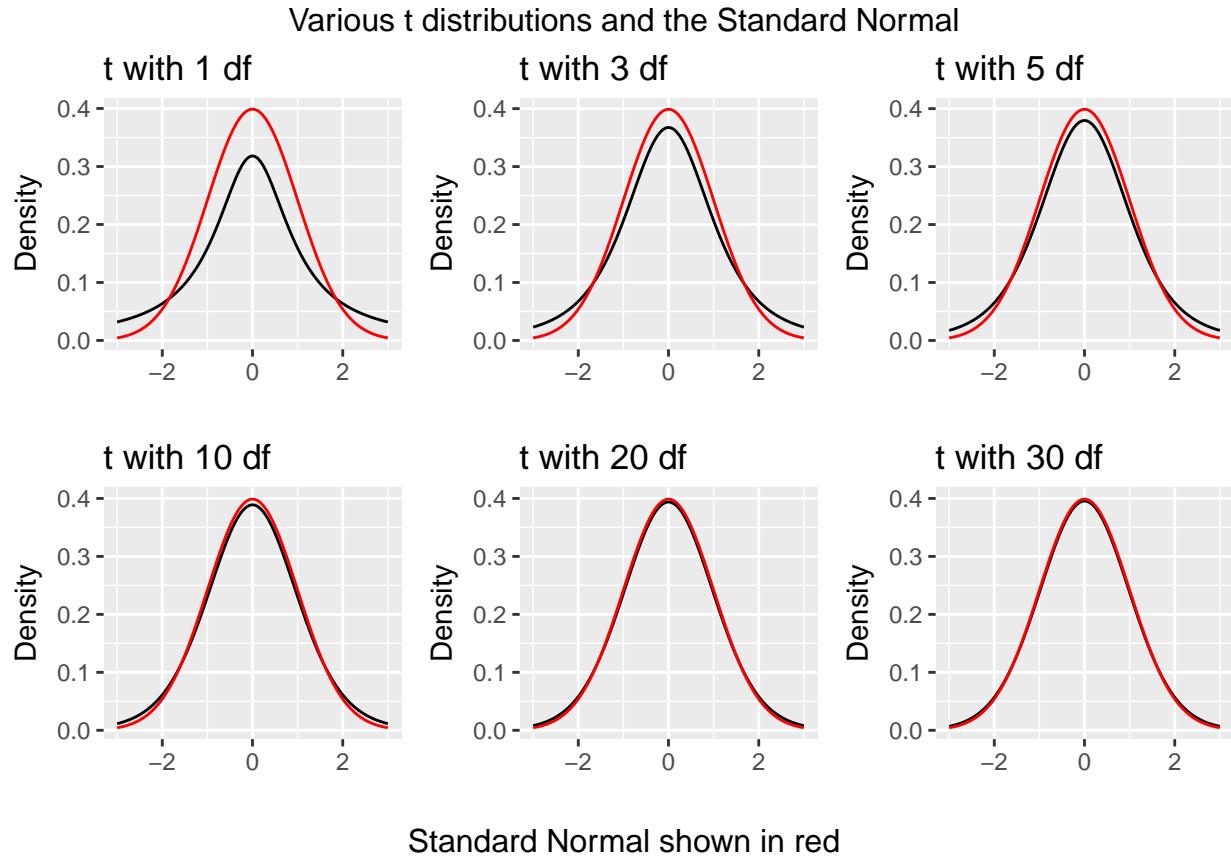
We obtain the relevant cutoff value in R by substituting in values for `alphaover2` and `n-1` into the following line of R code:

```
qt(alphaover2, df = n-1, lower.tail=FALSE)
```

### 19.5.2 Student's t distribution

Student's t distribution looks a lot like a Normal distribution, when the sample size is large. Unlike the normal distribution, which is specified by two parameters, the mean and the standard deviation, the t distribution is specified by one parameter, the degrees of freedom.

- t distributions with large numbers of degrees of freedom are more or less indistinguishable from the standard Normal distribution.
- t distributions with smaller degrees of freedom (say, with  $df < 30$ , in particular) are still symmetric, but are more outlier-prone than a Normal distribution



### 19.5.3 Building the CI “by hand” for the Serum Zinc data

In the serum zinc data, we observe the following results in our sample.

```
mosaic::favstats(serzinc$zinc)
```

	min	Q1	median	Q3	max	mean	sd	n	missing
	50	76	86	98	153	87.9	16	462	0

Suppose we wish to build a 90% confidence interval for the true mean serum zinc level across the entire population of teenage males. The confidence level will be 90%, or 0.90, and so the  $\alpha$  value, which is  $1 - \text{confidence} = 0.10$ .

So what we know going in is that:

- We want  $\alpha = 0.10$ , because we’re creating a 90% confidence interval.
- The sample size  $n = 462$  serum zinc measurements.
- The sample mean of those measurements,  $\bar{x} = 87.937$  micrograms per deciliter.
- The sample standard deviation of those measurements,  $s = 16.005$  micrograms per deciliter.
- As a result, our standard error of the sample mean is estimated well with  $s/\sqrt{n} = 16.005/\sqrt{462} = 0.745$ .

So now, we are ready to calculate our 90% confidence interval.

The two-sided  $100(1-\alpha)\%$  confidence interval (based on a  $t$  test) is:  $\bar{x} \pm t_{\alpha/2, n-1}(s/\sqrt{n})$ , or

- The 90% CI for  $\mu$  is thus  $87.937 \pm t_{0.10/2, 462-1} (0.745)$ 
  - To calculate the t cutoff value for  $\alpha = 0.10$  and  $n = 462$ , we use

```
qt(0.10/2, df = 462-1, lower.tail=FALSE) = 1.648
```

- So the 90% CI for  $\mu$  is  $87.937 \pm 1.648 \times 0.745$ , or
- $87.937 \pm 1.228$ , or
- $(86.71, 89.16)$

So, our 90% confidence interval for the true population mean serum zinc level, based on our sample of 462 patients, is  $(86.71, 89.16)$  micrograms per deciliter.

#### 19.5.4 Getting R to build a CI for the Serum Zinc data

Happily, R does all of this work, and with less inappropriate rounding.

```
t.test(serzinc$zinc, conf.level = 0.90, alternative = "two.sided")
```

One Sample t-test

```
data: serzinc$zinc
t = 100, df = 500, p-value <2e-16
alternative hypothesis: true mean is not equal to 0
90 percent confidence interval:
86.7 89.2
sample estimates:
mean of x
87.9
```

And again, our 90% confidence interval for the true population mean serum zinc level, based on our sample of 462 patients, is  $(86.7, 89.2)$  micrograms per deciliter<sup>1</sup>.

#### 19.5.5 Interpreting the Result

An appropriate interpretation of the 90% two-sided confidence interval above follows:

- $(86.71, 89.16)$  micrograms per deciliter is a 90% two-sided confidence interval for the population mean serum zinc level among teenage males.
- Our point estimate for the true population mean serum zinc level is 87.9. The values in the interval  $(86.71, 89.16)$  represent a reasonable range of estimates for the true population mean serum zinc level, and we are 90% confident that this method of creating a confidence interval will produce a result containing the true population mean serum zinc level.
- Were we to draw 100 samples of size 462 from the population described by this sample, and use each such sample to produce a confidence interval in this manner, approximately 90 of those confidence intervals would cover the true population mean serum zinc level.

#### 19.5.6 What if we want a 95% or 99% confidence interval instead?

The `t.test` function in R has an argument to specify the desired confidence level.

```
t.test(serzinc$zinc, conf.level = 0.95, alternative = "two.sided")
```

One Sample t-test

```
data: serzinc$zinc
```

---

<sup>1</sup>Since the measured zinc levels appear as integers, we should certainly not include any more than one additional significant figure in our confidence interval.

```
t = 100, df = 500, p-value <2e-16
alternative hypothesis: true mean is not equal to 0
95 percent confidence interval:
86.5 89.4
sample estimates:
mean of x
87.9
t.test(serzinc$zinc, conf.level = 0.99, alternative = "two.sided")
```

One Sample t-test

```
data: serzinc$zinc
t = 100, df = 500, p-value <2e-16
alternative hypothesis: true mean is not equal to 0
99 percent confidence interval:
86.0 89.9
sample estimates:
mean of x
87.9
```

Below, we see two-sided confidence intervals for various levels of  $\alpha$ .

Confidence Level	$\alpha$	Two-Sided Interval Estimate for Zinc Level Population Mean, $\mu$	Point Estimate for Zinc Level Population Mean, $\mu$
80% or 0.80	0.20	(87, 88.9)	87.9
90% or 0.90	0.10	(86.7, 89.2)	87.9
95% or 0.95	0.05	(86.5, 89.4)	87.9
99% or 0.99	0.01	(86, 89.9)	87.9

What happens to the width of the confidence interval in this table as the confidence level changes?

### 19.5.7 One-sided vs. Two-sided Confidence Intervals

In some situations, we are concerned with either an upper limit for the population mean  $\mu$  or a lower limit for  $\mu$ , but not both.

If we, as before, have a sample of size  $n$ , with sample mean  $\bar{x}$  and sample standard deviation  $s$ , then:

- The upper bound for a one-sided  $100(1-\alpha)\%$  confidence interval for the population mean is  $\mu \leq \bar{x} + t_{\alpha,n-1}(\frac{s}{\sqrt{n}})$ , with lower “bound”  $-\infty$ .
- The corresponding lower bound for a one-sided  $100(1 - \alpha)$  CI for  $\mu$  would be  $\mu \geq \bar{x} - t_{\alpha,n-1}(\frac{s}{\sqrt{n}})$ , with upper “bound”  $\infty$ .

### 19.5.8 Calculating a one-sided confidence interval for the population mean

```
t.test(serzinc$zinc, conf.level = 0.90, alternative = "greater")
```

One Sample t-test

```

data: serzinc$zinc
t = 100, df = 500, p-value <2e-16
alternative hypothesis: true mean is greater than 0
90 percent confidence interval:
 87 Inf
sample estimates:
mean of x
 87.9
t.test(serzinc$zinc, conf.level = 0.90, alternative = "less")

```

#### One Sample t-test

```

data: serzinc$zinc
t = 100, df = 500, p-value = 1
alternative hypothesis: true mean is less than 0
90 percent confidence interval:
-Inf 88.9
sample estimates:
mean of x
 87.9

```

### 19.5.9 Relationship between One-Sided and Two-Sided CIs

Note the relationship between the *two-sided* 80% confidence interval, and the *one-sided* 90% confidence intervals.

Confidence	$\alpha$	Type of Interval	Interval Estimate for Zinc Level
			Population Mean, $\mu$
80% (.80)	0.20	Two-Sided	(86.98, 88.89)
90% (.90)	0.10	One-Sided (Less Than)	$\mu < 88.89$ .
90% (.90)	0.10	One-Sided (Greater Than)	$\mu > 86.98$ .

Why does this happen? The 80% two-sided interval is placed so as to cut off the top 10% of the distribution with its upper bound, and the bottom 10% of the distribution with its lower bound. The 90% “less than” one-sided interval is placed so as to have its lower bound cut off the top 10% of the distribution.

The same issue appears when we consider two-sided 90% and one-sided 95% confidence intervals.

Confidence	$\alpha$	Type of Interval	Interval Estimate for Zinc Level
			Population Mean, $\mu$
90% (.90)	0.10	Two-Sided	(86.71, 89.16)
95% (.95)	0.05	One-Sided (Less Than)	$\mu < 89.16$ .
95% (.95)	0.05	One-Sided (Greater Than)	$\mu > 86.71$ .

Again, the 90% two-sided interval cuts off the top 5% and bottom 5% of the distribution with its bounds. The 95% “less than” one-sided interval also has its lower bound cut off the top 5% of the distribution.

### 19.5.10 Using the `broom` package with the t test

The `broom` package takes the messy output of built-in functions in R, such as `lm`, `t.test` or `wilcox.test`, and turns them into tidy data frames. A detailed description of the package and three of its key functions is found at <https://github.com/tidyverse/broom>.

For example, we can use the `tidy` function within `broom` to create a single-row tibble of the key results from a t test.

```
tt <- t.test(serzinc$zinc, conf.level = 0.95, alternative = "two.sided")
broom::tidy(tt)
```

```
estimate statistic p.value parameter conf.low conf.high
1     87.9      118       0      461     86.5     89.4
method alternative
1 One Sample t-test  two.sided
```

We can thus pull the endpoints of a 95% confidence interval directly from this output. `broom` also has a `glance` function, which returns the same information as `tidy` in the case of a t-test.

```
tt2 <- t.test(serzinc$zinc, conf.level = 0.90, alternative = "less")
broom::glance(tt2)
```

```
estimate statistic p.value parameter conf.low conf.high
1     87.9      118       1      461      -Inf     88.9
method alternative
1 One Sample t-test      less
```

## 19.6 Bootstrap Confidence Intervals for $\mu$

### 19.6.1 What is a Bootstrap and Why Should I Care?

The bootstrap (and in particular, what's known as bootstrap resampling) is a really good idea that you should know a little bit about. Good (2005) and Good and Hardin (2006) are excellent resources, for instance.

If we want to know how accurately a sample mean estimates the population mean, we would ideally like to take a very, very large sample, because if we did so, we could conclude with something that would eventually approach mathematical certainty that the sample mean would be very close to the population mean.

But we can rarely draw enormous samples. So what can we do?

### 19.6.2 Resampling is A Big Idea

One way to find out how precise our estimates are is to run them on multiple samples of the same size. This *resampling* approach was codified originally by Brad Efron in, for example, Efron (1979).

Oversimplifying a lot, the idea is that if we sample (with replacement) from our current sample, we can draw a new sample of the same size as our original.

- And if we repeat this many times, we can generate as many samples of, say, 462 zinc levels, as we like.
- Then we take these thousands of samples and calculate (for instance) the sample mean for each, and plot a histogram of those means.
- If we then cut off the top and bottom 5% of these sample means, we obtain a reasonable 90% confidence interval for the population mean.

### 19.6.3 When is a Bootstrap Confidence Interval for $\mu$ Reasonable?

The interval will be reasonable as long as we're willing to believe that:

- the original sample was a random sample (or at least a completely representative sample) from a population,
- and that the samples are independent of each other
- and that the samples are identically distributed (even though that distribution may not be Normal.)

A downside is that you and I will get (somewhat) different answers if we resample from the same data without setting the same random seed.

### 19.6.4 Bootstrap: Steps to estimate a confidence interval for $\mu$

To avoid the Normality assumption, and take advantage of modern computing power, we use R to obtain a bootstrap confidence interval for the population mean based on a sample.

What the computer does:

1. Resample the data with replacement, until it obtains a new sample that is equal in size to the original data set.
2. Calculates the statistic of interest (here, a sample mean.)
3. Repeat the steps above many times (the default is 1,000 using our approach) to obtain a set of 1,000 sample means.
4. Sort those 1,000 sample means in order, and estimate the 95% confidence interval for the population mean based on the middle 95% of the 1,000 bootstrap samples.
5. Send us a result, containing the sample mean, and a 95% confidence interval for the population mean

### 19.6.5 Using R to estimate a 90% CI for $\mu$ with the bootstrap

The command that we use to obtain a CI for  $\mu$  using the basic nonparametric bootstrap and without assuming a Normally distributed population, is `smean.cl.boot`, a part of the `Hmisc` package in R.

```
set.seed(431); Hmisc::smean.cl.boot(serzinc$zinc, B = 1000, conf.int = 0.90)
```

Mean	Lower	Upper
87.9	86.8	89.2

- Remember that the t-based 90% CI for  $\mu$  was (86.71, 89.16), according to the following output...

```
t.test(serzinc$zinc, conf.level = 0.90, alternative = "two.sided")
```

One Sample t-test

```
data: serzinc$zinc
t = 100, df = 500, p-value <2e-16
alternative hypothesis: true mean is not equal to 0
90 percent confidence interval:
86.7 89.2
sample estimates:
mean of x
87.9
```

### 19.6.6 Comparing Bootstrap and T-Based Confidence Intervals

- The `smean.cl.boot` function (unlike most R functions) deletes missing data automatically, as does the `smean.cl.normal` function, which produces the t-based confidence interval.

```
set.seed(431); Hmisc::smean.cl.boot(serzinc$zinc, B = 1000, conf.int = 0.90)
```

Mean	Lower	Upper
87.9	86.8	89.2

```
Hmisc::smean.cl.normal(serzinc$zinc, conf.int = 0.90)
```

Mean	Lower	Upper
87.9	86.7	89.2

Bootstrap resampling confidence intervals do not follow the general confidence interval strategy using a point estimate  $\pm$  a margin for error.

- A bootstrap interval is often asymmetric, and while it will generally have the point estimate (the sample mean) near its center, for highly skewed data, this will not necessarily be the case.
- We will usually use either 1,000 (the default) or 10,000 bootstrap replications for building confidence intervals – practically, it makes little difference.

### 19.6.7 90% CI for $\mu$ via bootstrap, changing minor details

Suppose we change the random seed that we set, or change the number (B) of desired bootstrap replications.

```
set.seed(431); Hmisc::smean.cl.boot(serzinc$zinc, B = 1000, conf.int = 0.90)
```

Mean	Lower	Upper
87.9	86.8	89.2

```
set.seed(431212); Hmisc::smean.cl.boot(serzinc$zinc, B = 1000, conf.int = 0.90)
```

Mean	Lower	Upper
87.9	86.7	89.2

```
set.seed(431212); Hmisc::smean.cl.boot(serzinc$zinc, B = 2000, conf.int = 0.90)
```

Mean	Lower	Upper
87.9	86.7	89.2

### 19.6.8 Bootstrap: Changing the Confidence Level

```
set.seed(431654); Hmisc::smean.cl.boot(serzinc$zinc, conf.int = 0.95, B = 1000)
```

Mean	Lower	Upper
87.9	86.4	89.3

```
set.seed(431321); Hmisc::smean.cl.boot(serzinc$zinc, conf.int = 0.99, B = 1000)
```

Mean	Lower	Upper
87.9	86.1	89.8

### 19.6.9 Bootstrap: Obtaining a One-sided Confidence Interval

If you want to estimate a one tailed confidence interval for the population mean using the bootstrap, then the procedure is as follows:

1. Determine  $\alpha$ , the significance level you want to use in your one-sided confidence interval. Remember that  $\alpha$  is 1 minus the confidence level. Let's assume we want a 90% one-sided interval, so  $\alpha = 0.10$ .
2. Double  $\alpha$  to determine the significance level we will use in the next step to fit a two-sided confidence interval.
3. Fit a two-sided confidence interval with confidence level  $100(1 - 2 * \alpha)$ . Let the bounds of this interval be  $(a, b)$ .
4. The one-sided (greater than) confidence interval will have  $a$  as its lower bound.
5. The one-sided (less than) confidence interval will have  $b$  as its upper bound.

Suppose that we want to find a 95% one-sided upper bound for the population mean serum zinc level among teenage males,  $\mu$ , using the bootstrap.

Since we want a 95% confidence interval, we have  $\alpha = 0.05$ . We double that to get  $\alpha = 0.10$ , which implies we need to instead fit a two-sided 90% confidence interval.

```
set.seed(43101); Hmisc::smean.cl.boot(serzinc$zinc, conf.int = 0.90, B = 1000)
```

Mean	Lower	Upper
87.9	86.7	89.3

Since the upper bound of this two-sided 90% CI is 89.27, that will also be the upper bound for a 95% one-sided CI.

### 19.6.10 Bootstrap CI for the Population Median

If we are willing to do a small amount of programming work in R, we can obtain bootstrap confidence intervals for other population parameters besides the mean. One statistic of common interest is the median. How do we find a confidence interval for the population median using a bootstrap approach? The easiest way I know of makes use of the `boot` package, as follows.

In step 1, we specify a new function to capture the medians from our sample.

```
f.median <- function(y, id)
{ median ( y[id]) }
```

In step 2, we summon the `boot` package and call the `boot.ci` function:

```
set.seed(431787)
boot.ci(boot (serzinc$zinc, f.median, 1000), conf=0.90, type="basic")
```

```
BOOTSTRAP CONFIDENCE INTERVAL CALCULATIONS
Based on 1000 bootstrap replicates
```

```
CALL :
boot.ci(boot.out = boot(serzinc$zinc, f.median, 1000), conf = 0.9,
type = "basic")
```

```
Intervals :
Level      Basic
90%   (84, 87 )
Calculations and Intervals on Original Scale
```

This yields a 90% confidence interval<sup>2</sup> for the population median serum zinc level.

Recall that the sample median for the serum zinc levels in our sample of 462 teenage males was 86 micrograms per deciliter.

### 19.6.11 Bootstrap CI for the IQR

If for some reason, we want to find a 95% confidence interval for the population value of the inter-quartile range via the bootstrap, we can do it.

```
IQR(serzinc$zinc)
```

```
[1] 22
f.IQR <- function(y, id)
{   IQR (y[id]) }

set.seed(431207); boot.ci(boot (serzinc$zinc, f.IQR, 1000),
                           conf=0.95, type="basic")
```

```
BOOTSTRAP CONFIDENCE INTERVAL CALCULATIONS
Based on 1000 bootstrap replicates
```

```
CALL :
boot.ci(boot.out = boot(serzinc$zinc, f.IQR, 1000), conf = 0.95,
         type = "basic")
```

```
Intervals :
Level      Basic
95%    (19, 24 )
Calculations and Intervals on Original Scale
```

### 19.6.12 Bootstrap Resampling: Advantages and Caveats

The bootstrap may seem like the solution to all estimation problems. In theory, we could use the same approach to find a confidence interval for any other parameter – it's not perfect, but it is very useful. Bootstrap procedures exist for virtually any statistical comparison - the t-test analog is just one of many possibilities, and bootstrap methods are rapidly gaining on more traditional approaches in the literature thanks mostly to faster computers.

The great advantage of the bootstrap is its relative simplicity, but don't forget that many of the original assumptions of the t-based confidence interval still hold.

- Using a bootstrap does eliminate the need to worry about the Normality assumption in small sample size settings, but it still requires independent and identically distributed samples from the population of interest.

The bootstrap produces clean and robust inferences (such as confidence intervals) in many tricky situations. It is still possible that the results can be both:

- **inaccurate** (i.e. they can include the true value of the unknown population mean less often than the stated confidence probability) and
- **imprecise** (i.e., they can include more extraneous values of the unknown population mean than is desirable).

---

<sup>2</sup>Actually, the boot.ci function can provide up to five different types of confidence interval (see the help file) if we change to type="all", and some of those other versions have attractive properties. However, we'll stick with the basic approach in 431.

## 19.7 Large-Sample Normal Approximation CIs for $\mu$

If we were in the position of knowing the standard deviation of the population of interest precisely<sup>3</sup>, we could use that information to build a  $100(1-\alpha)\%$  confidence interval using the Normal distribution, based on the sample mean  $\bar{x}$ , the sample size  $n$ , and the (known) population standard deviation  $\sigma$ .

When we have a large sample size (often as little as 60 observations), we can use this approach to get a very close approximation to the result we would get using the t distribution, and there are many settings where obtaining the Z test result is more appropriate in estimating more complicated parameters than the population mean.

### 19.7.1 The Large Sample Formula for the CI around $\mu$

The two-sided  $100(1-\alpha)\%$  confidence interval for a population mean  $\mu$  (based on the Normal distribution) is:

- The Lower Bound is  $\bar{x} - Z_{\alpha/2}(\sigma/\sqrt{n})$  and the Upper Bound is  $\bar{x} + Z_{\alpha/2}(\sigma/\sqrt{n})$

where  $Z_{\alpha/2}$  is the value that cuts off the top  $\alpha/2$  percent of the standard Normal distribution (the Normal distribution with mean 0 and standard deviation 1).

### 19.7.2 Obtaining the $Z_{\alpha/2}$ value using `qnorm`

We can obtain this cutoff value from R by substituting in the desired proportion for `alphaover2` into the `qnorm` function as follows:

```
qnorm(alphaover2, lower.tail=FALSE)
```

For example, if we are building a 95% confidence interval, we have  $100(1-\alpha) = 95$ , so that  $\alpha$  is 0.05, or 5%. This means that the cutoff value we need to find is  $Z_{0.05/2} = Z_{.025}$ , and this turns out to be 1.96.

```
qnorm(0.025, lower.tail=FALSE)
```

```
[1] 1.96
```

### 19.7.3 Commonly Used Cutoffs based on the Normal Distribution

- If we're building a two-sided 95% confidence interval, we'll use  $Z_{.025} = 1.96$
- For a two-sided 90% confidence interval, we use  $Z_{.05} = 1.645$
- For a two-sided 99% confidence interval, we use  $Z_{.005} = 2.576$
- For a two-sided 50% confidence interval, we use  $Z_{.25} = 0.67$
- For a two-sided 68% confidence interval, we use  $Z_{.16} = 0.99$

### 19.7.4 Lots of CIs use the Normal distribution

- The usual 95% confidence interval for large samples is an estimate  $\pm 2$  standard errors<sup>4</sup>.
- Also, from the Normal distribution, an estimate  $\pm 1$  standard error is a 68% confidence interval, and an estimate  $\pm 2/3$  of a standard error is a 50% confidence interval.
- A 50% interval is particularly easy to interpret because the true value should be inside the interval about as often as it is not.

<sup>3</sup>Practical applications usually demand a subtler approach, but this normal distribution-based approach can help us fix some key ideas

<sup>4</sup>The use of 2 standard errors for a confidence interval for a population mean is certainly reasonable whenever n is 60 or more. This is because the t distribution with 59 degrees of freedom has a 0.025 cutoff of 2.0, anyway.

- A 95% interval is thus about three times as wide as a 50% interval.
- In general, the larger the confidence required, the wider the interval will need to be.

### 19.7.5 Large-Sample Confidence Interval for Zinc Levels

Since we have a fairly large sample ( $n = 462$ ) in the `serzinc` data, we could consider using a large-sample approach (assuming the sample standard deviation is equal to the population standard deviation, and then using the Normal distribution) to estimate a confidence interval for the mean zinc levels in the population of all 15-17 year old males like those in our sample.

In the zinc levels within the `serzinc` data, we have

- a sample of  $n = 462$  observations
- with sample mean  $\bar{x} = 87.94$  and standard deviation  $s = 16$
- and suppose we want to, at first, find a 95% confidence interval, so  $\alpha = 0.05$

The 95% confidence interval is calculated as  $\bar{x} \pm Z_{\alpha/2}(\sigma/\sqrt{n})$ , and here we will assume that  $s = \sigma$  which may be reasonable with a fairly large sample size:

$$87.94 \pm (1.96)(16 / \sqrt{462}) = 87.94 \pm 1.46, \text{ or } (86.48, 89.4)$$

Our 95% confidence interval for the population mean is  $(86.48, 89.4) \mu\text{g/dl}$ . Were we to generate 100 such intervals, approximately 95 of those intervals would be expected to include the true mean of the entire population of 15-17 year old males like those in our sample.

### 19.7.6 Comparing Z and t-based Intervals for Serum Zinc

For the serum zinc data, we had  $n = 462$  observations in our sample.

Do the z-based and t-based confidence intervals differ much?

$\alpha$	Confidence Level	Confidence Interval	Method
0.05	95%	(86.48, 89.40)	Z (known $\sigma$ ; large $n$ )
0.05	95%	(86.47, 89.40)	t ( $\sigma$ unknown)
0.10	90%	(86.72, 89.16)	Z (known $\sigma$ ; large $n$ )
0.10	90%	(86.71, 89.16)	t ( $\sigma$ unknown)

### 19.7.7 One-Sided Confidence Intervals in Large Samples

The upper bound for a one-sided  $100(1-\alpha)\%$  confidence interval for the population mean is:

$$\mu \leq \bar{x} + Z_\alpha(\frac{\sigma}{\sqrt{n}}), \text{ with lower "bound" } -\infty.$$

The corresponding lower bound for a one-sided  $100(1 - \alpha)$  CI for  $\mu$  would be:

$$\mu \geq \bar{x} - Z_\alpha(\frac{\sigma}{\sqrt{n}}), \text{ with upper "bound" } \infty.$$

## 19.8 Wilcoxon Signed Rank Procedure for CIs

### 19.8.1 Confidence Intervals for the Median of a Population

It turns out to be difficult, without the bootstrap, to estimate an appropriate confidence interval for the median of a population, which might be an appealing thing to do, particularly if the sample data are clearly

not Normally distributed, so that a median seems like a better summary of the center of the data. Bootstrap procedures are available to perform the task.

The Wilcoxon signed rank approach can be used as an alternative to t-based procedures to build interval estimates for the population *pseudo-median* when the population cannot be assumed to follow a Normal distribution.

As it turns out, if you're willing to assume the population is **symmetric** (but not necessarily Normally distributed) then the pseudo-median is actually equal to the population median.

### 19.8.2 What is a Pseudo-Median?

The pseudo-median of a particular distribution G is the median of the distribution of  $(u + v)/2$ , where both u and v have the same distribution (G).

- If the distribution G is symmetric, then the pseudomedian is equal to the median.
- If the distribution is skewed, then the pseudomedian is not the same as the median.
- For any sample, the pseudomedian is defined as the median of all of the midpoints of pairs of observations in the sample.

### 19.8.3 Getting the Wilcoxon Signed Rank-based CI in R

```
wilcox.test(serzinc$zinc, conf.int=TRUE, conf.level=0.95)
```

```
Wilcoxon signed rank test with continuity correction

data: serzinc$zinc
V = 1e+05, p-value <2e-16
alternative hypothesis: true location is not equal to 0
95 percent confidence interval:
 86.0 88.5
sample estimates:
(pseudo)median
          87.5
```

### 19.8.4 Interpreting the Wilcoxon CI for the Population Median

If we're willing to believe the `zinc` levels come from a population with a symmetric distribution, the 95% Confidence Interval for the population median would be (86, 88.5)

For a non-symmetric population, this only applies to the *pseudo-median*.

Note that the pseudo-median (87.5) is actually closer here to the sample mean (86) than it is to the sample median (87.9).

### 19.8.5 Using the `broom` package with the Wilcoxon test

We can also use the `tidy` function within `broom` to create a single-row tibble of the key results from a Wilcoxon test.

```
wt <- wilcox.test(serzinc$zinc, conf.int=TRUE, conf.level=0.95)
broom::tidy(wt)
```

```

estimate statistic p.value conf.low conf.high
1      87.5    106953   2e-77       86       88.5
                                              method alternative
1 Wilcoxon signed rank test with continuity correction two.sided

```

## 19.9 General Advice

We have described four different approaches to estimating a confidence interval for the center of a distribution of quantitative data.

1. The most commonly used approach uses the  $t$  distribution to estimate a confidence interval for a population/process mean. This requires some extra assumptions, most particularly that the underlying distribution of the population values is at least approximately Normally distributed.
2. A more modern and very general approach uses the idea of the bootstrap to estimate a confidence for a population/process parameter, which could be a mean, median or other summary statistic. The bootstrap, and the underlying notion of *resampling* is an important idea that lets us avoid some of the assumptions (in particular Normality) that are required by other methods. Bootstrap confidence intervals involve random sampling, so that the actual values obtained will differ a bit across replications.
3. A third approach makes more substantial assumptions - it uses the Normal distribution rather than a  $t$ , and assumes (among other things) very large samples. For estimating a single mean, we'll rarely use this, but for estimating more complex parameters, particularly in Part C when discussing modeling, we will occasionally use this approach.
4. Finally, the Wilcoxon signed-rank method is one of a number of inferential tools which transform the data to their *ranks* before estimating a confidence interval. This avoids some assumptions, but yields inferences about a less-familiar parameter - the pseudo-median.

Most of the time, the **bootstrap** provides an adequate solution when estimating a confidence interval to describe the population value of a parameter (mean or median, most commonly) from a distribution, when our data consists of a single sample of quantitative information.

## Chapter 20

# Confidence Intervals from Two Paired Samples of Quantitative Data

Here, we'll consider the problem of estimating a confidence interval to describe the difference in population means (or medians) based on a comparison of two samples of quantitative data, gathered using a matched pairs design. Specifically, we'll use as our example the Lead in the Blood of Children study, described in Section 17.

Recall that in that study, we measured blood lead content, in mg/dl, for 33 matched pairs of children, one of which was exposed (had a parent working in a battery factory) and the other of which was control (no parent in the battery factory, but matched to the exposed child by age, exposure to traffic and neighborhood). We then created a variable called `leaddiff` which contained the (exposed - control) differences within each pair.

`bloodlead`

```
# A tibble: 33 x 4
  pair exposed control leaddiff
  <fctr>   <int>    <int>    <int>
1 P01      38       16      22
2 P02      23       18       5
3 P03      41       18      23
4 P04      18       24      -6
5 P05      37       19      18
6 P06      36       11      25
7 P07      23       10      13
8 P08      62       15      47
9 P09      31       16      15
10 P10     34       18      16
# ... with 23 more rows
```

### 20.1 t-based CI for Population Mean of Paired Differences, $\mu_d$ .

In R, there are at least three different methods for obtaining the t-based confidence interval for the population difference in means between paired samples. They are all mathematically identical. The key idea is to calculate the paired differences (exposed - control, for example) in each pair, and then treat the result as if it were a single sample and apply the methods discussed in Section 19.

### 20.1.1 Method 1

We can use the single-sample approach, applied to the variable containing the paired differences. Let's build a **90%** two-sided confidence interval for the population mean of the difference in blood lead content across all possible pairs of an exposed (parent works in a lead-based industry) and a control (parent does not) child,  $\mu_d$ .

```
t.test(bloodlead$leaddir, conf.level = 0.90, alt = "two.sided")
```

One Sample t-test

```
data: bloodlead$leaddir
t = 6, df = 30, p-value = 2e-06
alternative hypothesis: true mean is not equal to 0
90 percent confidence interval:
 11.3 20.6
sample estimates:
mean of x
 16
```

The 90% confidence interval is (11.29, 20.65) according to this t-based procedure. An appropriate interpretation of the 90% two-sided confidence interval would be:

- (11.29, 20.65) milligrams per deciliter is a 90% two-sided confidence interval for the population mean difference in blood lead content between exposed and control children.
- Our point estimate for the true population difference in mean blood lead content is 15.97 mg.dl. The values in the interval (11.29, 20.65) mg/dl represent a reasonable range of estimates for the true population difference in mean blood lead content, and we are 90% confident that this method of creating a confidence interval will produce a result containing the true population mean difference.
- Were we to draw 100 samples of 33 matched pairs from the population described by this sample, and use each such sample to produce a confidence interval in this manner, approximately 90 of those confidence intervals would cover the true population mean difference in blood lead content levels.

### 20.1.2 Method 2

Or, we can apply the single-sample approach to a calculated difference in blood lead content between the exposed and control groups. Here, we'll get a **95%** two-sided confidence interval for  $\mu_d$ , instead of the 90% interval we obtained above.

```
t.test(bloodlead$exposed - bloodlead$control,
       conf.level = 0.95, alt = "two.sided")
```

One Sample t-test

```
data: bloodlead$exposed - bloodlead$control
t = 6, df = 30, p-value = 2e-06
alternative hypothesis: true mean is not equal to 0
95 percent confidence interval:
 10.3 21.6
sample estimates:
mean of x
 16
```

### 20.1.3 Method 3

Or, we can provide R with two separate samples (unaffected and affected) and specify that the samples are paired. Here, we'll get a **99% one-sided** confidence interval (lower bound) for  $\mu_d$ , the population mean difference in blood lead content.

```
t.test(bloodlead$exposed, bloodlead$control, conf.level = 0.99,
       paired = TRUE, alt = "greater")
```

```
Paired t-test

data: bloodlead$exposed and bloodlead$control
t = 6, df = 30, p-value = 1e-06
alternative hypothesis: true difference in means is greater than 0
99 percent confidence interval:
 9.21 Inf
sample estimates:
mean of the differences
               16
```

Again, the three different methods using `t.test` for paired samples will all produce identical results if we feed them the same confidence level and type of interval (two-sided, greater than or less than).

### 20.1.4 Assumptions

If we are building a confidence interval for the mean  $\mu$  of a population or process based on a sample of observations drawn from that population, then we must pay close attention to the assumptions of those procedures. The confidence interval procedure for the population mean  $\mu$  using the t distribution assumes that:

1. We want to estimate the population mean  $\mu$ .
2. We have drawn a sample of observations at random from the population of interest.
3. The sampled observations are drawn from the population independently and have identical distributions.
4. The population follows a Normal distribution. At the very least, the sample itself is approximately Normal.

### 20.1.5 Using `broom` for a t test using paired samples

The `broom` package places the results of a t test, among other things, into a tidy data frame.

```
broom::tidy(t.test(bloodlead$exposed - bloodlead$control,
                   conf.level = 0.95, alt = "two.sided"))
```

	estimate	statistic	p.value	parameter	conf.low	conf.high
1	16	5.78	2.04e-06	32	10.3	21.6
				method	alternative	
1	One Sample t-test			two.sided		

## 20.2 Bootstrap CI for mean difference using paired samples

The same bootstrap approach is used for paired differences as for a single sample. We again use the `smean.cl.boot()` function in the `Hmisc` package to obtain bootstrap confidence intervals for the population

mean,  $\mu_d$ , of the paired differences in blood lead content.

```
set.seed(431555)
Hmisc::smean.cl.boot(bloodlead$leaddir, conf.int = 0.95, B = 1000)
```

Mean	Lower	Upper
16.0	10.8	21.3

Note that in this case, the confidence interval for the difference in means is a bit less wide than the 95% confidence interval generated by the t test, which was (10.34, 21.59). It's common for the bootstrap to produce a narrower range (i.e. an apparently more precise estimate) for the population mean, but it's not automatic that the endpoints from the bootstrap will be inside those provided by the t test, either.

For example, this bootstrap CI doesn't contain the t-test based interval, since its upper bound exceeds that of the t-based interval:

```
set.seed(4310003)
Hmisc::smean.cl.boot(bloodlead$leaddir, conf.int = 0.95, B = 1000)
```

Mean	Lower	Upper
16.0	11.0	21.8

And neither does this one, which actually covers a wider range than the t-based interval.

```
set.seed(4310018)
Hmisc::smean.cl.boot(bloodlead$leaddir, conf.int = 0.95, B = 1000)
```

Mean	Lower	Upper
16.0	10.3	21.8

This demonstration aside, the appropriate thing to do when applying the bootstrap to specify a confidence interval is select a seed and the number ( $B = 1,000$  or  $10,000$ , usually) of desired bootstrap replications, then run the bootstrap just once and move on, rather than repeating the process multiple times looking for a particular result.

### 20.2.1 Assumptions

The bootstrap confidence interval procedure for the population mean (or median) assumes that:

1. We want to estimate the population mean  $\mu$  (or the population median).
2. We have drawn a sample of observations at random from the population of interest.
3. The sampled observations are drawn from the population independently and have identical distributions.
4. We are willing to put up with the fact that different people (not using the same random seed) will get somewhat different confidence interval estimates using the same data.

As we've seen, a major part of the bootstrap's appeal is the ability to relax some assumptions.

## 20.3 Wilcoxon Signed Rank-based CI for paired samples

We could also use the Wilcoxon signed rank procedure to generate a CI for the pseudo-median of the paired differences.

```
wilcox.test(bloodlead$leaddir,
            conf.int = TRUE,
            conf.level = 0.90,
            exact = FALSE)
```

```
Wilcoxon signed rank test with continuity correction
```

```
data: bloodlead$leaddir
V = 500, p-value = 1e-05
alternative hypothesis: true location is not equal to 0
90 percent confidence interval:
 11.0 20.5
sample estimates:
(pseudo)median
 15.5
```

As in the one sample case, we can revise this code slightly to specify a different confidence level, or gather a one-sided rather than a two-sided confidence interval.

### 20.3.1 Assumptions

The Wilcoxon signed rank confidence interval procedure assumes that:

1. We want to estimate the population **median**.
2. We have drawn a sample of observations at random from the population of interest.
3. The sampled observations are drawn from the population independently and have identical distributions.
4. The population follows a symmetric distribution. At the very least, the sample itself shows no substantial skew, so that the sample pseudo-median is a reasonable estimate for the population median.

### 20.3.2 Using **broom** and the **tidy** function with a Wilcoxon procedure

```
broom::tidy(wilcox.test(bloodlead$leaddir,
                       conf.int = TRUE,
                       conf.level = 0.90,
                       exact = FALSE))

estimate statistic p.value conf.low conf.high
1      15.5        499 1.15e-05       11       20.5
                                              method alternative
1 Wilcoxon signed rank test with continuity correction two.sided
```

## 20.4 Choosing a Confidence Interval Approach

Suppose we want to find a confidence interval for the mean of a population,  $\mu$ , or, the population mean difference  $\mu_d$  between two populations based on matched pairs.

1. If we are willing to assume that the population distribution is **Normal**
  - and that the population SD  $\sigma$  is known, we can use a Z-based CI.
  - and the population SD  $\sigma$  isn't known, we use a t-based CI.
2. If we are **unwilling** to assume that the population is Normal,
  - use a **bootstrap** procedure to get a CI for the population mean, or even the median
  - but are willing to assume the population is symmetric, consider a **Wilcoxon signed rank** procedure to get a CI for the median, rather than the mean.

The two methods you'll use most often are the bootstrap (especially if the data don't appear to be at least pretty well fit by a Normal model) and the t-based confidence intervals (if the data do appear to fit a Normal model well.)



# Chapter 21

## Confidence Intervals from Two Independent Samples of Quantitative Data

Here, we'll consider the problem of estimating a confidence interval to describe the difference in population means (or medians) based on a comparison of two samples of quantitative data, gathered using an independent samples design. Specifically, we'll use as our example the randomized controlled trial of Ibuprofen in Sepsis patients, as described in Section @ref(Sepsis\_RCT).

In that trial, 300 patients meeting specific criteria (including elevated temperature) for a diagnosis of sepsis were randomly assigned to either the Ibuprofen group (150 patients) and 150 to the Placebo group. Group information (our exposure) is contained in the `treat` variable. The key outcome of interest to us was `temp_drop`, the change in body temperature (in °C) from baseline to 2 hours later, so that positive numbers indicate drops in temperature (a good outcome.)

`sepsis`

```
# A tibble: 300 x 6
  id      treat    race apache temp_0 temp_drop
  <chr>   <fctr>  <fctr> <int>   <dbl>     <dbl>
1 S002   Ibuprofen AfricanA     14   38.7      1.4
2 S004   Ibuprofen White       3    38.3      0.4
3 S005   Placebo    White     5    38.6      0.0
4 S006   Ibuprofen White     13   38.2     -0.2
5 S009   Ibuprofen White     25   38.2      0.6
6 S011   Ibuprofen White     21   38.1     -0.4
7 S012   Placebo    White     14   38.6     -0.1
8 S014   Placebo    White     23   37.9      0.3
9 S016   Placebo    White     16   38.1      0.1
10 S020  Ibuprofen Other      20   39.2      1.5
# ... with 290 more rows
```

## 21.1 t-based CI for population mean difference $\mu_1 - \mu_2$ from Independent Samples

### 21.1.1 The Welch t procedure

The default confidence interval based on the t test for independent samples in R uses something called the Welch test, in which the two populations being compared are not assumed to have the same variance. Each population is assumed to follow a Normal distribution.

```
t.test(sepsis$temp_drop ~ sepsis$treat, conf.level = 0.90, alt = "two.sided")
```

Welch Two Sample t-test

```
data: sepsis$temp_drop by sepsis$treat
t = 4, df = 300, p-value = 3e-05
alternative hypothesis: true difference in means is not equal to 0
90 percent confidence interval:
 0.191 0.432
sample estimates:
mean in group Ibuprofen   mean in group Placebo
      0.464                  0.153
```

### 21.1.2 The Pooled t procedure

The most commonly used t-procedure for building a confidence interval assumes not only that each of the two populations being compared follows a Normal distribution, but also that they have the same population variance. This is the pooled t-test, and it is what people usually mean when they describe a two-sample t test.

```
t.test(sepsis$temp_drop ~ sepsis$treat, conf.level = 0.90, alt = "two.sided", var.equal = TRUE)
```

Two Sample t-test

```
data: sepsis$temp_drop by sepsis$treat
t = 4, df = 300, p-value = 3e-05
alternative hypothesis: true difference in means is not equal to 0
90 percent confidence interval:
 0.191 0.432
sample estimates:
mean in group Ibuprofen   mean in group Placebo
      0.464                  0.153
```

### 21.1.3 Using linear regression to obtain a pooled t confidence interval

A linear regression model, using the same outcome and predictor (group) as the pooled t procedure, produces the same confidence interval, again, under the assumption that the two populations we are comparing follow a Normal distribution with the same (population) variance.

```
model1 <- lm(temp_drop ~ treat, data = sepsis)
model1
```

```
Call:
lm(formula = temp_drop ~ treat, data = sepsis)
```

```
Coefficients:
(Intercept)  treatPlacebo
      0.464       -0.311
confint(model1, level = 0.90)
```

	5 %	95 %
(Intercept)	0.379	0.549
treatPlacebo	-0.432	-0.191

We see that our point estimate from the linear regression model is that the difference in `temp_drop` is -0.311, where Ibuprofen subjects have higher `temp_drop` values than do Placebo subjects, and that the 90% confidence interval for this difference ranges from -0.432 to -0.191.

We can obtain a t-based confidence interval for each of the parameter estimates in a linear model directly using `confint`. Linear models usually summarize only the estimate and standard error. Remember that a reasonable approximation in large samples to a 95% confidence interval for a regression estimate (slope or intercept) can be obtained from  $\text{estimate} \pm 2 * \text{standard error}$ .

```
summary(model1)
```

```
Call:
lm(formula = temp_drop ~ treat, data = sepsis)

Residuals:
    Min      1Q  Median      3Q     Max 
-2.8527 -0.3640 -0.0527  0.3473  2.6360 

Coefficients:
            Estimate Std. Error t value Pr(>|t|)    
(Intercept)  0.4640     0.0516   8.99 < 2e-16 ***
treatPlacebo -0.3113     0.0730  -4.27  2.7e-05 ***
---
Signif. codes:  0 '***' 0.001 '**' 0.01 '*' 0.05 '.' 0.1 ' ' 1
```

Residual standard error: 0.632 on 298 degrees of freedom  
Multiple R-squared: 0.0575, Adjusted R-squared: 0.0544  
F-statistic: 18.2 on 1 and 298 DF, p-value: 2.68e-05

So, in the case of the `treatPlacebo` estimate, we can obtain an approximate 95% confidence interval with  $-0.311 \pm 2 \times 0.073$  or (-0.457, -0.165). Compare this to the 95% confidence interval available from the model directly, shown below, and you'll see only a small difference.

```
confint(model1, level = 0.95)
```

	2.5 %	97.5 %
(Intercept)	0.362	0.566
treatPlacebo	-0.455	-0.168

## 21.2 Bootstrap CI for $\mu_1 - \mu_2$ from Independent Samples

The `bootdif` function contained in the `Love-boost.R` script, that we will use in this setting is a slightly edited version of the function at <http://biostat.mc.vanderbilt.edu/wiki/Main/BootstrapMeansSoftware>. Note that this approach uses a comma to separate the outcome variable (here, `temp_drop`) from the variable identifying the exposure groups (here, `treat`).

```
set.seed(431212)
bootdif(sepsis$temp_drop, sepsis$treat, conf.level = 0.90)
```

Mean Difference	0.05	0.95
-0.311	-0.431	-0.197

## 21.3 Wilcoxon Rank Sum-based CI from Independent Samples

As in the one-sample case, a rank-based alternative attributed to Wilcoxon (and sometimes to Mann and Whitney) provides a two-sample comparison of the pseudomedians in the two `treat` groups in terms of `temp_drop`. This is called a **rank sum** test, rather than the **signed rank** test for a single sample. Here's the resulting 90% confidence interval.

```
wilcox.test(sepsis$temp_drop ~ sepsis$treat,
            conf.int = TRUE, conf.level = 0.90,
            alt = "two.sided")
```

```
Wilcoxon rank sum test with continuity correction

data: sepsis$temp_drop by sepsis$treat
W = 10000, p-value = 7e-06
alternative hypothesis: true location shift is not equal to 0
90 percent confidence interval:
 0.2 0.4
sample estimates:
difference in location
 0.3
```

## 21.4 Using the `tidy` function from `broom` for t and Wilcoxon procedures

The `tidy` function is again available to us in dealing with a t-test or Wilcoxon rank sum test.

```
broom::tidy(t.test(sepsis$temp_drop ~ sepsis$treat,
                   conf.level = 0.90,
                   alt = "two.sided"))

estimate estimate1 estimate2 statistic p.value parameter conf.low
1    0.311     0.464     0.153      4.27 2.71e-05      288     0.191
conf.high                         method alternative
1    0.432 Welch Two Sample t-test   two.sided

broom::tidy(wilcox.test(sepsis$temp_drop ~ sepsis$treat,
                       conf.int = TRUE,
```

```
conf.level = 0.90,
alt = "two.sided"))

estimate statistic p.value conf.low conf.high
1      0.3     14614 7.28e-06      0.2       0.4
method alternative
1 Wilcoxon rank sum test with continuity correction two.sided
```

We can also use `broom` functions to place the elements of the linear model `model1` into a tidy data frame. This provides the estimate of the Placebo-Ibuprofen difference, and its standard error, which we could use to formulate a confidence interval.

```
broom::tidy(model1)

term estimate std.error statistic p.value
1 (Intercept) 0.464    0.0516     8.99 2.91e-17
2 treatPlacebo -0.311   0.0730    -4.27 2.68e-05

rm(model1)
```



## Chapter 22

# Hypothesis Testing of a Population Mean

Hypothesis testing or significance testing uses sample data to attempt to reject the hypothesis that nothing interesting is happening – that is, to reject the notion that chance alone can explain the sample results<sup>1</sup>. We can, in many settings, use confidence intervals to summarize the results, as well, and confidence intervals and hypothesis tests are closely connected. Significance tests have a valuable role to play, but this role is more limited than many scientists realize, and it is unfortunate that tests are widely misused.

In particular, it's worth stressing that:

- **A significant effect is not necessarily the same thing as an interesting effect.** For example, results calculated from large samples are nearly always “significant” even when the effects are quite small in magnitude. Before doing a test, always ask if the effect is large enough to be of any practical interest. If not, why do the test?
- **A non-significant effect is not necessarily the same thing as no difference.** A large effect of real practical interest may still produce a non-significant result simply because the sample is too small.
- **There are assumptions behind all statistical inferences.** Checking assumptions is crucial to validating the inference made by any test or confidence interval.

### 22.1 Five Steps Required in Completing a Hypothesis Test

1. Specify the null hypothesis,  $H_0$  (which usually indicates that there is no difference or no association between the results in various groups of subjects)
2. Specify the research or alternative hypothesis,  $H_A$ , sometimes called  $H_1$  (which usually indicates that there is some difference or some association between the results in those same groups of subjects).
3. Specify the test procedure or test statistic to be used to make inferences to the population based on sample data. Here is where we usually specify  $\alpha$ , the probability of incorrectly rejecting  $H_0$  that we are willing to accept. In the absence of other information, we often use  $\alpha = 0.05$
4. Obtain the data, and summarize it to obtain the relevant test statistic, which gets summarized as a  $p$  value.
5. Use the  $p$  value to either
  - **reject  $H_0$**  in favor of the alternative  $H_A$  (concluding that there is a statistically significant difference/association at the  $\alpha$  significance level) or

---

<sup>1</sup>Some of this is adapted from @GoodHardin, and @Utts1999

- **retain  $H_0$**  (and conclude that there is no statistically significant difference/association at the  $\alpha$  significance level)

## 22.2 Hypothesis Testing for the Serum Zinc Example

We previously studied serum zinc levels in micrograms per deciliter gathered for a sample of 462 males aged 15-17. “Typical” values are said to be 70-110  $\mu\text{g}/\text{dl}$ . Suppose we want to conduct a hypothesis test to see whether our observed zinc values are statistically significantly different from a value we hypothesize might be a reasonable guess for the population as a whole, let’s specify **90  $\mu\text{g}/\text{dl}$** .

### 22.2.1 Our Research Question

Is there reasonable evidence, based on this sample of 462 males aged 15-17, for us to conclude that the population of males aged 15-17 from which this sample was drawn will have a mean serum zinc level that is statistically significantly different from 90  $\mu\text{g}/\text{dl}$ , the midpoint of the range of “typical” values in the general population?

### 22.3 Step 1. Specify the null hypothesis

Our population parameter  $\mu$  = the mean serum zinc level (in  $\mu\text{g}/\text{dl}$ ) across the entire population of males aged 15-17.

- We’re testing whether  $\mu$  is significantly different from a pre-specified value, 90  $\mu\text{g}/\text{dl}$ .
- To do this, we apply our pre-specified value in our null hypothesis, so  $H_0 : \mu = 90$ .

### 22.4 Step 2. Specify the research hypothesis

The research hypothesis is the opposite of the null hypothesis. Here, that’s just  $H_A : \mu \neq 90$ .

### 22.5 Step 3. Specify the test procedure

Again, we’ll opt for the usual  $\alpha = 0.05$ . The main procedures for this one-sample setting include three of the four options we used with paired samples, specifically a one-sample t-test, a one-sample Wilcoxon signed rank test, or a bootstrap confidence interval.

- Remember our  $H_0$  specifies  $\mu = 90$ , rather than  $\mu = 0$ , as is often the case.

### 22.6 Step 4. Obtain the $p$ value and/or confidence interval

Of course, we’ve already collected the data. If we’re willing to assume the 462 serum zinc levels we have are a random (or sufficiently representative) sample of the population of interest, and that the data were gathered in such a way that each sample is independent of every other sample, and identically distributed, then our methods might work.

### 22.6.1 Assuming a Normal distribution in the population yields a t test.

```
t.test(serzinc$zinc)
```

One Sample t-test

```
data: serzinc$zinc
t = 100, df = 500, p-value <2e-16
alternative hypothesis: true mean is not equal to 0
95 percent confidence interval:
 86.5 89.4
sample estimates:
mean of x
 87.9
```

Whoops! This is **WRONG**. Remember that we need to specify that our alternative hypothesis is that the true mean is not equal to 90, not to zero. To change this, we specify our null hypothesis `mu` value in the `t.test` function, as follows...

```
t.test(serzinc$zinc, mu=90)
```

One Sample t-test

```
data: serzinc$zinc
t = -3, df = 500, p-value = 0.006
alternative hypothesis: true mean is not equal to 90
95 percent confidence interval:
 86.5 89.4
sample estimates:
mean of x
 87.9
```

You'll note that the only changes here are in the  $t$  statistic,  $p$  value and alternative hypothesis. The degrees of freedom, confidence interval and sample mean are unchanged.

So the correct  $p$  value from the  $t$  test would be 0.006, which is less than our pre-specified  $\alpha$  of 0.05, and so we'd reject  $H_0$  and conclude that the population mean serum zinc level is statistically significantly different from 90.

- Notice that we would come to the same conclusion using the confidence interval. Specifically, using a 5% significance level (i.e. a 95% confidence level) a reasonable range for the true value of the population mean is entirely below 90 – it's (86.5, 89.4). So if 90 is not in the reasonable range, we'd reject  $H_0 : \mu = 90$ .

### 22.6.2 Using `broom` to tidy the results of our t test

```
broom::tidy(t.test(serzinc$zinc, mu=90))
```

	estimate	statistic	p.value	parameter	conf.low	conf.high
1	87.9	-2.77	0.00583	461	86.5	89.4
				method	alternative	
1	One Sample t-test			two.sided		

We can use the `tidy` function within the `broom` package to summarize the results of a t test, just as we did with a t-based confidence interval.

### 22.6.3 Wilcoxon signed rank test (doesn't require Normal assumption).

```
wilcox.test(serzinc$zinc, mu=90, conf.int=TRUE, exact = FALSE)
```

```
Wilcoxon signed rank test with continuity correction

data: serzinc$zinc
V = 40000, p-value = 3e-04
alternative hypothesis: true location is not equal to 90
95 percent confidence interval:
 85.5 88.5
sample estimates:
(pseudo)median
 87
```

Using the Wilcoxon signed rank test, we obtain a two-sided  $p$  value of 0.0003, which is far less than our pre-specified  $\alpha$  of 0.05, so we would, again, reject  $H_0 : \mu = 90$ .

- Again, the confidence interval suggests that the reasonable range for the population pseudomedian does not contain 90, so we'd reject  $H_0 : \mu = 90$  by that standard, too.
- We can again use the `tidy` function from the `broom` package to summarize the results of the Wilcoxon signed rank test.

```
broom::tidy(wilcox.test(serzinc$zinc, mu=90, conf.int = TRUE, exact = FALSE))
```

```
estimate statistic p.value conf.low conf.high
1       87      41613 0.000335     85.5      88.5
                                              method alternative
1 Wilcoxon signed rank test with continuity correction two.sided
```

### 22.6.4 Bootstrap Confidence Interval

```
set.seed(43123)
Hmisc::smean.cl.boot(serzinc$zinc)
```

Mean	Lower	Upper
87.9	86.6	89.4

The 95% confidence interval using the bootstrap procedure, again, does not include 90, so we would reject  $H_0 : \mu = 90$ , in favor of the alternative hypothesis  $H_A : \mu \neq 90$ .

## 22.7 Step 5. Reject or Retain $H_0$ and Draw Conclusions

Using any of these procedures, we would conclude that the null hypothesis (that the true mean serum zinc level for this population is 90  $\mu\text{g}/\text{dl}$ ) is not tenable, and that it should be rejected at the 5% significance level. The smaller the  $p$  value, the stronger is the evidence that the null hypothesis is incorrect, and in this case, we have some fairly tiny  $p$  values.

Of course, the confidence intervals suggest that the population mean is reasonably close to 90, and so the difference we can detect (using a fairly large sample of 462 subjects) may not be a clinically meaningful one.

## 22.8 A One-Sided Test of a Single Sample: What R Reports

Let's walk through a one-sided t test based on a single sample, including a one-sided 90% confidence interval. For instance, suppose we want to test whether the population (of males aged 15-17) has a mean serum zinc level that is statistically significantly **less than** 90  $\mu\text{g}/\text{dl}$ , based on the sample of 462 males aged 15-17 that we discussed earlier.

```
t.test(serzinc$zinc, mu = 90, conf = 0.90, alt="less")
```

```
One Sample t-test

data: serzinc$zinc
t = -3, df = 500, p-value = 0.003
alternative hypothesis: true mean is less than 90
90 percent confidence interval:
-Inf 88.9
sample estimates:
mean of x
87.9
```

Here's a brief summary of what R is calculating

1. A specification of the group being studied – here the **zinc** results
2. A specification as to which alternative hypothesis is being tested
  - Note that we are trying to see here if the population mean is less than 90, not 0.
  - here we have a one-sided, specifically a “less than” alternative hypothesis, and it means that we have the following null and alternative hypotheses,  $H_0 : \mu \geq 90$  and  $H_A : \mu < 90$ , where  $\mu =$  population mean serum zinc level
3. The point estimate (sample mean) of the population mean serum zinc level
  - The sample mean is given as 87.94, so it's at least possible that the true population mean could be less than 90.
4. A 90% confidence interval for the population mean serum zinc level
  - This is a one-sided confidence interval, done with 90% confidence.
  - Since it's one-sided, and we have a “less than” alternative hypothesis, we will only be specifying an upper bound for the population mean.
    - If we had a “greater than” alternative, we would specify a lower bound, instead.
  - The upper bound from a  $100(1-\alpha)\%$  one-sided confidence interval for a population mean using the t distribution is  $\bar{x} \pm t_{\alpha,n-1}(s/\sqrt{n})$
  - As before, we sample n observations from the population, and  $\bar{x} =$  the sample mean,  $s =$  the sample standard deviation, and  $\alpha$  is the significance level (so that  $100[1-\alpha]$  is the confidence level, and  $t_{\alpha,n-1}$  is the upper tail cutoff value for a probability of  $\alpha$  for the t distribution with  $n - 1$  degrees of freedom).
5. R then calculates ...
  - the sample mean of the  $n = 462$  serum zinc levels ( $\bar{x} = 87.9372$ ), and
  - the sample standard deviation of the paired differences (which turns out to be  $s = 16.0047$ ).
  - In order to find a 90% confidence interval, we would need  $\alpha = 0.10$ , so we use the appropriate tool in R to find the t cutoff for  $\alpha = 0.10$  with appropriate degrees of freedom ( $n - 1 = 462 - 1$  or 461).

```
qt(0.10, 461, lower.tail=FALSE)
```

```
[1] 1.28
```

So  $t_{\alpha,n-1} = t_{0.10,461} = 1.283$ , and we can now complete the calculation.

$$\bar{x} \pm t_{\alpha,n-1} (s / \sqrt{n}) = 87.9372 + 1.283(16.0047 / \sqrt{462}) = 87.9372 + 0.9553 = 88.893$$

6. A t statistic, degrees of freedom and  $p$  value, based on the data that test the null and alternative hypotheses under study

- Here, this is  $t = -2.7703$ ,  $df = 461$ ,  $p$ -value = 0.002913.
- The t statistic again is the sample mean minus the null hypothesized value of the population mean, all divided by the standard error of the sample mean (i.e. the sample standard deviation divided by the square root of the sample size.)
- Or, in mathematical terms,  $t = (\bar{x} - \mu_0) / (s / \sqrt{n}) = (87.9372 - 90) / (16.0047 / \sqrt{462}) = (-2.0628) / 0.7446 = -2.77$ .
- We can interpret the t statistic as the “number of standard errors the sample mean is away from the null hypothesized value of the population mean”.
- The degrees of freedom for a single sample comparison like this is just the number of observations minus 1. Here, we have 462 serum zinc results; 461 degrees of freedom.
- Given the test statistic,  $t = -2.77$ , and the degrees of freedom  $n-1 = 461$ , R can now calculate a  $p$  value, specifically the probability (given that  $H_0$  is true) of observing a result as much in favor of the alternative hypothesis HA as these data suggest.
- We want a one-sided  $p$  value here, since we have a one-sided alternative hypothesis (i.e. a “less than” alternative).
- Find the probability of getting a result this small or smaller (since we have a “less than” alternative, if it was a “greater than” alternative, we’d find the probability of a result this large or larger) as follows...

```
pt(-2.77, df=461, lower.tail=TRUE)
```

```
[1] 0.00292
```

# Chapter 23

## Type I and Type II Error: Power and Confidence

Once we know how unlikely the results would have been if the null hypothesis were true, we must make one of two choices:

1. The  $p$  value is not small enough to convincingly rule out chance. Therefore, we cannot reject the null hypothesis as an explanation for the results.
2. The  $p$  value was small enough to convincingly rule out chance. We reject the null hypothesis and accept the alternative hypothesis.

Making choice 2 is equivalent to declaring that the result is statistically significant. We can rephrase the two choices as:

1. There is no statistically significant difference or relationship in the data.
2. There is a statistically significant difference or relationship in the data.

How small must the  $p$  value be in order to rule out the null hypothesis? The standard choice is 5%. This standardization has advantages and disadvantages<sup>1</sup>, and it is not compulsory. It is simply a convention that has become accepted over the years, and there are many situations for which a 5% cutoff may be unwise. While it does give a specific, objectively chosen level to keep in mind, it suggests a rather mindless cutpoint having nothing to do with the importance of the decision nor the costs or losses associated with outcomes.

### 23.1 The Courtroom Analogy

Consider the analogy of the jury in a courtroom.

1. The evidence is not strong enough to convincingly rule out that the defendant is innocent. Therefore, we cannot reject the null hypothesis, or innocence of the defendant.
2. The evidence was strong enough that we are willing to rule out the possibility that an innocent person (as stated in the null hypothesis) produced the observed data. We reject the null hypothesis, that the defendant is innocent, and assert the alternative hypothesis.

Consistent with our thinking in hypothesis testing, in many cases we would not accept the hypothesis that the defendant is innocent. We would simply conclude that the evidence was not strong enough to rule out the possibility of innocence.

---

<sup>1</sup>Ingelfinger JA, Mosteller F, Thibodeau LA and Ware JH (1987) Biostatistics in Clinical Medicine, 2nd Edition, New York: MacMillan. pp. 156-157.

The  $p$  value is the probability of getting a result as extreme or more extreme than the one observed if the proposed null hypothesis is true. Notice that it is not valid to actually accept that the null hypothesis is true. To do so would be to say that we are essentially convinced that chance alone produced the observed results – a common mistake.

## 23.2 Significance vs. Importance

Remember that a statistically significant relationship or difference does not necessarily mean an important one. A result that is significant in the statistical meaning of the word may not be significant clinically. Statistical significance is a technical term. Findings can be both statistically significant and practically significant or either or neither.

When we have very large samples, we may find small differences statistically significant even though they have no clinical importance. At the other extreme, with small samples, even large differences will often not be significant at the levels usually required to recognize the difference as real. We must distinguish between statistical and practical/clinical significance.

## 23.3 Errors in Hypothesis Testing

In testing hypotheses, there are two potential decisions and each one brings with it the possibility that a mistake has been made.

Let's use the courtroom analogy. Here are the potential choices and associated potential errors. Although the seriousness of errors depends on the seriousness of the crime and punishment, the potential error for choice 2 is usually more serious.

1. We cannot rule out that the defendant is innocent, so (s)he is set free without penalty.
  - Potential Error: A criminal has been erroneously freed.
2. We believe that there is enough evidence to conclude that the defendant is guilty.
  - Potential Error: An innocent person is convicted / penalized and a guilty person remains free.

As another example, consider being tested for disease. Most tests for diseases are not 100% accurate. The lab technician or physician must make a choice:

1. In the opinion of the medical practitioner, you are healthy. The test result was weak enough to be called “negative” for the disease.
  - Potential Error: You are actually diseased but have been told you are not. This is called a **false negative**.
2. In the opinion of the medical practitioner, you are diseased. The test results were strong enough to be called “positive” for the disease.
  - Potential Error: You are actually healthy but have been told you are diseased. This is called a **false positive**.

## 23.4 The Two Types of Hypothesis Testing Errors

	H <sub>A</sub> is true	H <sub>0</sub> is true
Test Rejects H <sub>0</sub>	Correct Decision	Type I Error (False Positive)
Test Retains H <sub>0</sub>	Type II Error (False Negative)	Correct Decision

- A Type I error can only be made if the null hypothesis is actually true.

- A Type II error can only be made if the alternative hypothesis is actually true.

## 23.5 The Significance Level, $\alpha$ , is the Probability of a Type I Error

If the null hypothesis is true, the  $p$  value is the probability of making an error by choosing the alternative hypothesis instead. Alpha ( $\alpha$ ) is defined as the probability of concluding significance [rejection of  $H_0$ ] when there isn't (and  $H_0$  is true, making a Type I error), also called the significance level, so that  $100(1-\alpha)$  is the confidence level – the probability of correctly concluding that there is no difference (retaining  $H_0$ ) when  $H_0$  is true.

## 23.6 The Probability of avoiding a Type II Error is called Power, symbolized $1-\beta$

A Type II error is made if the alternative hypothesis is true, but you fail to choose it. The probability depends on exactly which part of the alternative hypothesis is true, so that computing the probability of making a Type II error is not feasible. The power of a test is the probability of making the correct decision when the alternative hypothesis is true. Beta ( $\beta$ ) is defined as the probability of concluding that there was no difference, when in fact there was one (a Type II error). Power is then just  $1 - \beta$ , the probability of concluding that there was a difference, when, in fact, there was one.

Traditionally, people like the power of a test to be at least 80%, meaning that  $\beta$  is at most 0.20. Often, I'll be arguing for 90% as a minimum power requirement, or we'll be presenting a range of power calculations for a variety of sample size choices.

## 23.7 Incorporating the Costs of Various Types of Errors

Which error is more serious in medical testing, where we think of our  $H_0$ : patient is healthy vs.  $H_A$ : disease is present?

It depends on the disease and on the consequences of a negative or positive test result. A false negative in a screening test for cancer could lead to a fatal delay in treatment, whereas a false positive would probably lead to a retest. A more troublesome example occurs in testing for an infectious disease. Inevitably, there is a trade-off between the two types of errors. It all depends on the consequences.

It would be nice if we could specify the probability that we were making an error with each potential decision. We could then weigh the consequence of the error against its probability. Unfortunately, in most cases, we can only specify the conditional probability of making a Type I error, given that the null hypothesis is true.

In deciding whether to reject a null hypothesis, we will need to consider the consequences of the two potential types of errors. If a Type I error is very serious, then you should reject the null hypothesis only if the  $p$  value is very small. Conversely, if a Type II error is more serious, you should be willing to reject the null hypothesis with a larger  $p$  value, perhaps 0.10 or 0.20, instead of 0.05.

## 23.8 Relation of $\alpha$ and $\beta$ to Error Types

- $\alpha$  is the probability of rejecting  $H_0$  when  $H_0$  is true.
  - So  $1 - \alpha$ , the confidence level, is the probability of retaining  $H_0$  when that's the right thing to do.
- $\beta$  is the probability of retaining  $H_0$  when  $H_A$  is true.
  - So  $1 - \beta$ , the power, is the probability of rejecting  $H_0$  when that's the right thing to do.

-	$H_A$ is True	$H_0$ is True
Test Rejects $H_0$	Correct Decision ( $1 - \beta$ )	Type I Error ( $\alpha$ )
Test Retains $H_0$	Type II Error ( $\beta$ )	Correct Decision ( $1 - \alpha$ )

## 23.9 Power and Sample Size Calculations

- For most statistical tests, it is theoretically possible to estimate the power of the test in the design stage, (before any data are collected) for various sample sizes, so we can hone in on a sample size choice which will enable us to collect data only on as many subjects as are truly necessary.
- A power calculation is likely the most common element of a scientific grant proposal on which a statistician is consulted. This is a fine idea in theory, but in practice...
- The tests that have power calculations worked out in intensive detail using R are mostly those with more substantial assumptions. Examples include t tests that assume population normality, common population variance and balanced designs in the independent samples setting, or paired t tests that assume population normality in the paired samples setting.
- These power calculations are also usually based on tests rather than confidence intervals, which would be much more useful in most settings. Simulation is your friend here.
- Even more unfortunately, this process of doing power and related calculations is **far more of an art than a science**.
- As a result, the value of many power calculations is negligible, since the assumptions being made are so arbitrary and poorly connected to real data.
- On several occasions, I have stood in front of a large audience of medical statisticians actively engaged in clinical trials and other studies that require power calculations for funding. When I ask for a show of hands of people who have had power calculations prior to such a study whose assumptions matched the eventual data perfectly, I get lots of laughs. It doesn't happen.
- Even the underlying framework that assumes a power of 80% with a significance level of 5% is sufficient for most studies is pretty silly.

All that said, I feel obliged to show you some examples of power calculations done using R, and provide some insight on how to make some of the key assumptions in a way that won't alert reviewers too much to the silliness of the enterprise.

## 23.10 Sample Size and Power Considerations for a Single-Sample t test

For a t test, R can estimate any one of the following elements, given the other four, using the `power.t.test` command, for either a one-tailed or two-tailed single-sample t test...

- $n$  = the sample size
- $\delta$  = delta = the true difference in population means between the null hypothesis value and a particular alternative
- $s$  =  $sd$  = the true standard deviation of the population
- $\alpha$  = `sig.level` = the significance level for the test (maximum acceptable risk of Type I error)
- $1 - \beta$  = power = the power of the t test to detect the effect of size  $\delta$

### 23.10.1 A Toy Example

Suppose that in a recent health survey, the average beef consumption in the U.S. per person was 90 pounds per year. Suppose you are planning a new study to see if beef consumption levels have changed. You plan to take a random sample of 25 people to build your new estimate, and test whether the current pounds of beef consumed per year is 90. Suppose you want to do a two-sided (two-tailed) test at 95% confidence (so  $\alpha = 0.05$ ), and that you expect that the true difference will need to be at least  $\delta = 5$  pounds (i.e. 85 or less or 95 or more) in order for the result to be of any real, practical interest. Suppose also that you are willing to assume that the true standard deviation of the measurements in the population is 10 pounds.

That is, of course, a lot to suppose.

Now, we want to know what power the proposed experiment will have to detect a change of 5 pounds (or more) away from the original 90 pounds, with these specifications, and how tweaking these specifications will affect the power of the study.

So, we have -  $n = 25$  data points to be collected -  $\delta = 5$  pounds is the minimum clinically meaningful effect size -  $s = 10$  is the assumed population standard deviation, in pounds per year -  $\alpha$  is 0.05, and we'll do a two-sided test

### 23.10.2 Using the `power.t.test` function

```
power.t.test(n = 25, delta = 5, sd = 10, sig.level = 0.05,
             type="one.sample", alternative="two.sided")
```

```
One-sample t test power calculation

      n = 25
      delta = 5
      sd = 10
      sig.level = 0.05
      power = 0.67
   alternative = two.sided
```

So, under this study design, we would expect to detect an effect of size  $\delta = 5$  pounds with just under 67% power, i.e. with a probability of incorrect retention of  $H_0$  of just about 1/3. Most of the time, we'd like to improve this power, and to do so, we'd need to adjust our assumptions.

### 23.10.3 Changing Assumptions in a Power Calculation

We made assumptions about the sample size  $n$ , the minimum clinically meaningful effect size (change in the population mean)  $\delta$ , the population standard deviation  $s$ , and the significance level  $\alpha$ , not to mention decisions about the test, like that we'd do a one-sample t test, rather than another sort of test for a single sample, and that we'd do a two-tailed, or two-sided test. Often, these assumptions are tweaked a bit to make the power look more like what a reviewer/funder is hoping to see.

### 23.10.4 Increasing the Sample Size, absent other changes, will Increase the Power

Suppose, we committed to using more resources and gathering data from 40 subjects instead of the 25 we assumed initially – what effect would this have on our power?

```
power.t.test(n = 40, delta = 5, sd = 10, sig.level = 0.05,
             type="one.sample", alternative="two.sided")
```

One-sample t test power calculation

```
n = 40
delta = 5
sd = 10
sig.level = 0.05
power = 0.869
alternative = two.sided
```

With more samples, we should have a more powerful test, able to detect the difference with greater probability. In fact, a sample of 40 paired differences yields 87% power. As it turns out, we would need at least 44 observations with this scenario to get to 90% power, as shown in the calculation below, which puts the power in, but leaves out the sample size.

```
power.t.test(power=0.9, delta = 5, sd = 10, sig.level = 0.05,
             type="one.sample", alternative="two.sided")
```

One-sample t test power calculation

```
n = 44
delta = 5
sd = 10
sig.level = 0.05
power = 0.9
alternative = two.sided
```

We see that we would need at least 44 observations to achieve 90% power. Note: we always round the sample size up in doing a power calculation – if this calculation had actually suggested  $n = 43.1$  paired differences were needed, we would still have rounded up to 44.

### 23.10.5 Increasing the Effect Size, absent other changes, will increase the Power

A larger effect should be easier to detect. If we go back to our original calculation, which had 67% power to detect an effect of size  $\delta = 5$ , and now change the desired effect size to  $\delta = 6$  pounds (i.e. a value of 84 or less or 96 or more), we should obtain a more powerful design.

```
power.t.test(n = 25, delta = 6, sd = 10, sig.level = 0.05,
             type="one.sample", alternative="two.sided")
```

One-sample t test power calculation

```
n = 25
delta = 6
sd = 10
sig.level = 0.05
power = 0.821
alternative = two.sided
```

We see that this change in effect size from 5 to 6, leaving everything else the same, increases our power from 67% to 82%. To reach 90% power, we'd need to increase the effect size we were trying to detect to at least

6.76 pounds.

```
power.t.test(n = 25, power = 0.9, sd = 10, sig.level = 0.05,
             type="one.sample", alternative="two.sided")
```

One-sample t test power calculation

```
n = 25
delta = 6.76
sd = 10
sig.level = 0.05
power = 0.9
alternative = two.sided
```

- Again, note that I am rounding up here.
- Using  $\delta = 6.75$  would not quite make it to 90.00% power.
- Using  $\delta = 6.76$  guarantees that the power will be 90% or more, and not just round up to 90%..

### 23.10.6 Decreasing the Standard Deviation, absent other changes, will increase the Power

The choice of standard deviation is usually motivated by a pilot study, or else pulled out of thin air - it's relatively easy to convince yourself that the true standard deviation might be a little smaller than you'd guessed initially. Let's see what happens to the power if we reduce the sample standard deviation from 10 pounds to 9. This should make the effect of 5 pounds easier to detect, because it will have smaller variation associated with it.

```
power.t.test(n = 25, delta = 5, sd = 9, sig.level = 0.05,
             type="one.sample", alternative="two.sided")
```

One-sample t test power calculation

```
n = 25
delta = 5
sd = 9
sig.level = 0.05
power = 0.76
alternative = two.sided
```

This change in standard deviation from 10 to 9, leaving everything else the same, increases our power from 67% to nearly 76%. To reach 90% power, we'd need to decrease the standard deviation of the population paired differences to no more than 7.39 pounds.

```
power.t.test(n = 25, delta = 5, sd = NULL, power = 0.9, sig.level = 0.05,
             type="one.sample", alternative="two.sided")
```

One-sample t test power calculation

```
n = 25
delta = 5
sd = 7.4
sig.level = 0.05
power = 0.9
```

```
alternative = two.sided
```

Note I am rounding down here.

- Using  $s = 7.4$  pounds would not quite make it to 90.00% power.

Note also that in order to get R to treat the  $sd$  as unknown, I must specify it as `NULL` in the formula...

### 23.10.7 Tolerating a Larger $\alpha$ (Significance Level), without other changes, increases Power

We can trade off some of our Type II error (lack of power) for Type I error. If we are willing to trade off some Type I error (as described by the  $\alpha$ ), we can improve the power. For instance, suppose we decided to run the original test with 90% confidence.

```
power.t.test(n = 25, delta = 5, sd = 10, sig.level = 0.1,
              type="one.sample", alternative="two.sided")
```

```
One-sample t test power calculation
```

```
n = 25
delta = 5
sd = 10
sig.level = 0.1
power = 0.783
alternative = two.sided
```

The calculation suggests that our power would thus increase from 67% to just over 78%.

# Chapter 24

## Comparing Two Means Using Paired Samples

In this section, we apply several methods of testing the null hypothesis that two populations have the same distribution of a quantitative variable. In particular, we'll focus on the comparison of means using paired sample t tests, signed rank tests, and bootstrap approaches. Our example comes from the Lead in the Blood of Children study, described in Section 17 and then developed further (including confidence intervals) in Section 20.

Recall that in that study, we measured blood lead content, in mg/dl, for 33 matched pairs of children, one of which was exposed (had a parent working in a battery factory) and the other of which was control (no parent in the battery factory, but matched to the exposed child by age, exposure to traffic and neighborhood). We then created a variable called `leaddiff` which contained the (exposed - control) differences within each pair.

`bloodlead`

```
# A tibble: 33 x 4
  pair exposed control leaddiff
  <fctr> <int>    <int>    <int>
1 P01      38      16     22
2 P02      23      18      5
3 P03      41      18     23
4 P04      18      24     -6
5 P05      37      19     18
6 P06      36      11     25
7 P07      23      10     13
8 P08      62      15     47
9 P09      31      16     15
10 P10     34      18     16
# ... with 23 more rows
```

### 24.1 Specifying A Two-Sample Study Design

These questions will help specify the details of the study design involved in any comparison of means.

1. What is the outcome under study?
2. What are the (in this case, two) treatment/exposure groups?
3. Were the data collected using matched / paired samples or independent samples?

4. Are the data a random sample from the population(s) of interest? Or is there at least a reasonable argument for generalizing from the sample to the population(s)?
5. What is the significance level (or, the confidence level) we require here?
6. Are we doing one-sided or two-sided testing/confidence interval generation?
7. If we have paired samples, did pairing help reduce nuisance variation?
8. If we have paired samples, what does the distribution of sample paired differences tell us about which inferential procedure to use?
9. If we have independent samples, what does the distribution of each individual sample tell us about which inferential procedure to use?

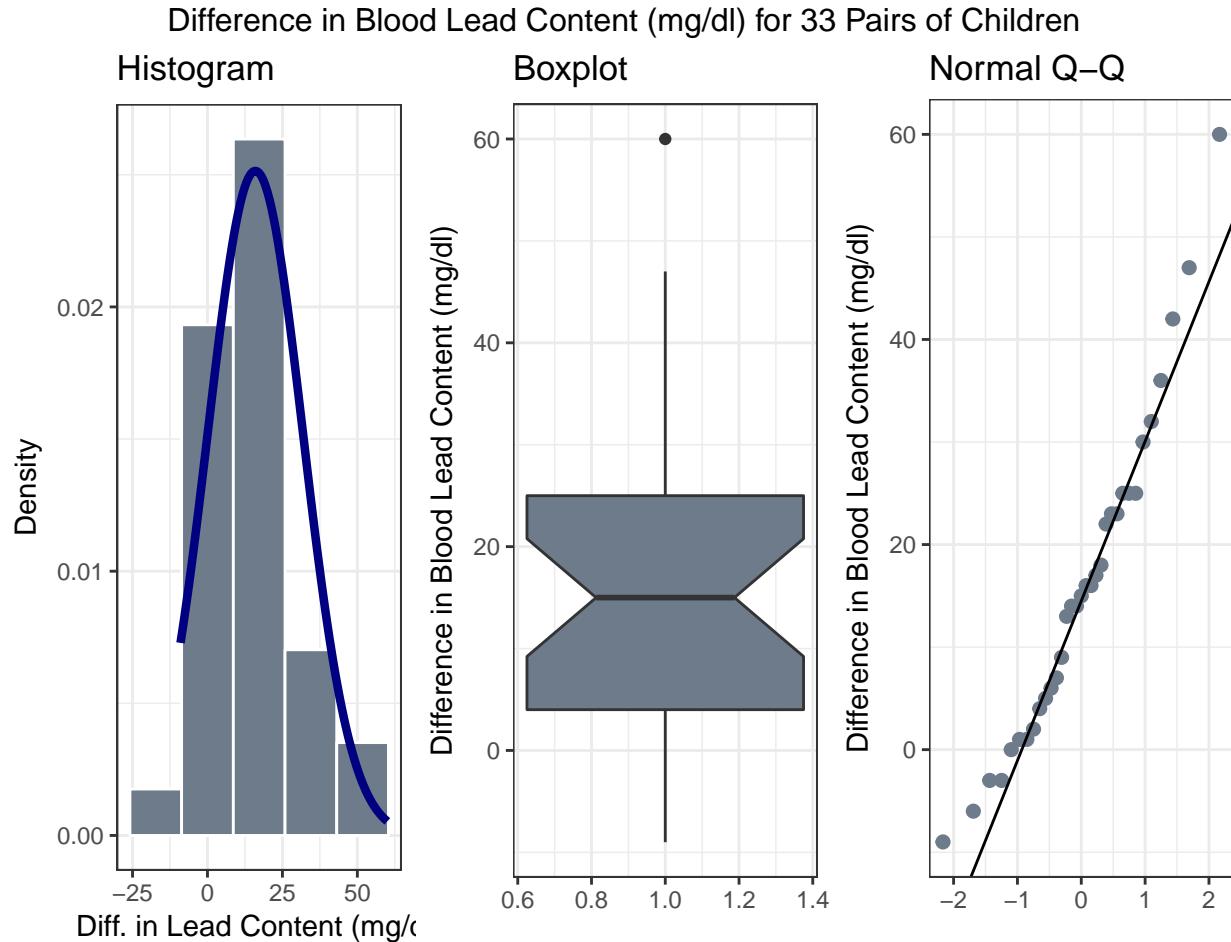
#### 24.1.1 For the bloodlead study

1. The outcome is blood lead content in mg/dl.
2. The groups are **exposed** (had a parent working in a battery factory) and **control** (no parent in the battery factory, but matched to the exposed child by age, exposure to traffic and neighborhood) children.
3. The data were collected using matched samples. The pairs of subjects are matched by age, exposure to traffic and neighborhood.
4. The data aren't a random sample of the population of interest, but we will assume for now that there's no serious issue with representing that population.
5. We'll use a 10% significance level (or 90% confidence level) in this setting.
6. We'll use a two-sided testing and confidence interval approach.

To answer question 7 (did pairing help reduce nuisance variation), we return to Section 17 where we saw that:

- The stronger the Pearson correlation coefficient, the more helpful pairing will be.
- Here, a straight line model using the control child's blood lead content accounts for about 3% of the variation in blood lead content in the exposed child.
- So, as it turns out, pairing will have only a modest impact here on the inferences we draw in the study.

To address question 8, we'll need to look at the data - specifically the paired differences. Repeating a panel of plots from Section 17, we will see that the paired differences appear to follow a Normal distribution, at least approximately (there's a single outlier, but with 33 pairs, that's not much of a concern, and the data are basically symmetric), so that a t-based procedure may be appropriate, or, at least, we'd expect to see similar results with t and bootstrap procedures.



Of course, question 9 doesn't apply here, because we have paired, and not independent samples.

## 24.2 Hypothesis Testing for the Blood Lead Example

### 24.2.1 Our Research Question

Is there reasonable evidence, based on these paired samples of 33 exposed and 33 control children, for us to conclude that the population of children similar to those in the exposed group will have a distribution of blood lead content that is statistically significantly different from the population of children similar to those in the control group. In other words, if we generated the population of all exposed-control differences across the entire population of such pairs, would that distribution of paired differences be centered at zero (indicating no difference in the means)?

Again, the key idea is to calculate the paired differences (exposed - control, for example) in each pair, and then treat the result as if it were a single sample and apply the methods discussed in Section 22.

### 24.2.2 Specify the null hypothesis

Our null hypothesis here is that the population (true) mean blood lead content in the exposed group is the same as the population mean blood lead content in the control group plus a constant value (which we'll symbolize with  $\Delta_0$  which is most often taken to be zero. Since we have paired samples, we can instead

describe this hypothesis in terms of the difference between exposed and control within each pair. So, our null hypothesis can be written either as:

$$H_0 : \mu_{Exposed} = \mu_{Control} + \Delta_0$$

where  $\Delta_0$  is a constant, usually taken to be zero, or

$$H_0 : \mu_{Exposed - Control} = \Delta_0,$$

where, again,  $\Delta_0$  is usually zero.

We will generally take this latter approach, where the population mean of the paired differences (here, exposed - control, but we could have just as easily selected control - exposed: the order is arbitrary so long as we are consistent) is compared to a constant value, usually 0.

For the `bloodlead` example, our population parameter  $\mu_{Exposed - Control}$  = the mean difference in blood lead content between the exposed and control groups (in mg/dl) across the entire population.

- We're testing whether  $\mu$  is significantly different from a pre-specified value, 0 mg/dl.

### 24.2.3 Specify the research hypothesis

The research hypothesis is that the population mean of the exposed - control differences is not equal to our constant value  $\Delta_0$ .

$$H_A : \mu_{Exposed - Control} \neq \Delta_0,$$

For the `bloodlead` example, we have  $H_A : \mu_{Exposed - Control} \neq 0$ .

### 24.2.4 Specify the test procedure and $\alpha$

As we've seen in Section 20, there are several ways to build a confidence interval to address these hypotheses, and each of those approaches provides information about a related hypothesis test. This includes several methods for obtaining a paired t test, plus a Wilcoxon signed rank test, and a bootstrap comparison of means (or medians, etc.) using paired samples. We'll specify an  $\alpha$  value of .10 here, indicating a 10% significance level (and 90% confidence level.)

### 24.2.5 Calculate the test statistic and $p$ value

For the paired t test and Wilcoxon signed rank test, Section 20 demonstrated the relevant R code for the `bloodlead` example to obtain  $p$  values. For the bootstrap procedure, we again build a confidence interval. We repeat that work below.

### 24.2.6 Draw a conclusion

As we've seen, we use the  $p$  value to either

- **reject**  $H_0$  in favor of the alternative  $H_A$  (concluding that there is a statistically significant difference/association at the  $\alpha$  significance level) if the  $p$  value is less than our desired  $\alpha$  or
- **retain**  $H_0$  (and conclude that there is no statistically significant difference/association at the  $\alpha$  significance level) if the  $p$  value is greater than or equal to  $\alpha$ .

## 24.3 Assuming a Normal distribution in the population of paired differences yields a paired t test.

```
t.test(bloodlead$exposed - bloodlead$control, conf = 0.90, alt = "two.sided")
```

One Sample t-test

```
data: bloodlead$exposed - bloodlead$control
t = 6, df = 30, p-value = 2e-06
alternative hypothesis: true mean is not equal to 0
90 percent confidence interval:
11.3 20.6
sample estimates:
mean of x
16
```

The t test statistic here is 6, based on 30 degrees of freedom, and this yields a  $p$  value of  $2.036e-06$  or  $2.036 \times 10^{-6}$ , or  $p = .000002036$ . This  $p$  value is certainly less than our pre-specified  $\alpha$  of 0.10, and so we'd reject  $H_0$  and conclude that the population mean of the exposed-control paired differences is statistically significantly different from 0.

- Notice that we would come to the same conclusion using the confidence interval. Specifically, using a 10% significance level (i.e. a 90% confidence level) a reasonable range for the true value of the population mean is entirely above 0 – it's (11.3, 20.6). So if 0 is not in the 90% confidence interval, we'd reject  $H_0 : \mu_{Exposed-Control} = 0$  at the 10% significance level.

### 24.3.1 Assumptions of the paired t test

We must be willing to believe that

1. the paired differences data are a random (or failing that, representative) sample from the population of interest, and
2. that the samples were drawn independently, from an identical population distribution

regardless of what testing procedure we use. For the paired t test, we must also assume that:

3. the paired differences come from a Normally distributed population.

### 24.3.2 Using broom to tidy the paired t test

```
broom::tidy(t.test(bloodlead$exposed - bloodlead$control,
conf = 0.90, alt = "two.sided"))
```

```
estimate statistic p.value parameter conf.low conf.high
1       16      5.78 2.04e-06       32      11.3      20.6
method alternative
1 One Sample t-test   two.sided
```

We can use the `tidy` function within the `broom` package to summarize the results of a t test, just as we did with a t-based confidence interval.

### 24.3.3 Calculation Details: The Paired t test

The paired t test is calculated using:

- $\bar{d}$ , the sample mean of the paired differences,
- the null hypothesized value  $\Delta_0$  for the differences (which is usually 0),
- $s_d$ , the sample standard deviation, and
- $n$ , the sample size (number of pairs).

We have

$$t = \frac{\bar{d} - \Delta_0}{s_d / \sqrt{n}}$$

which is then compared to a  $t$  distribution with  $n - 1$  degrees of freedom to obtain a  $p$  value.

Wikipedia's page on Student's t test is a good resource for these calculations.

## 24.4 The Bootstrap Approach: Build a Confidence Interval

The same bootstrap approach is used for paired differences as for a single sample. We again use the `smean.cl.boot()` function in the `Hmisc` package to obtain bootstrap confidence intervals for the population mean,  $\mu_d$ , of the paired differences in blood lead content.

```
set.seed(431888)
Hmisc::smean.cl.boot(bloodlead$exposed - bloodlead$control, conf.int = 0.90, B = 1000)
```

Mean	Lower	Upper
16.0	11.6	20.6

Since 0 is not contained in this 90% confidence interval, we reject the null hypothesis (that the mean of the paired differences in the population is zero) at the 10% significance level, so we know that  $p < 0.10$ .

### 24.4.1 Assumptions of the paired samples bootstrap procedure

We still must be willing to believe that

1. the paired differences data are a random (or failing that, representative) sample from the population of interest, and
2. that the samples were drawn independently, from an identical population distribution

regardless of what testing procedure we use. But, for the bootstrap, we do not also need to assume Normality of the population distribution of paired differences.

## 24.5 The Wilcoxon signed rank test (doesn't require Normal assumption).

We could also use the Wilcoxon signed rank procedure to generate a CI for the pseudo-median of the paired differences.

```
wilcox.test(bloodlead$leaddirf,
            conf.int = TRUE,
```

```
conf.level = 0.90,
exact = FALSE)
```

```
Wilcoxon signed rank test with continuity correction

data: bloodlead$leaddir
V = 500, p-value = 1e-05
alternative hypothesis: true location is not equal to 0
90 percent confidence interval:
11.0 20.5
sample estimates:
(pseudo)median
15.5
```

Using the Wilcoxon signed rank test, we obtain a two-sided  $p$  value of  $1.155 \times 10^{-5}$ , which is far less than our pre-specified  $\alpha$  of 0.10, so we would, again, reject  $H_0 : \mu_{Exposed-Control} = 0$  at the 10% significance level.

- We can again use the `tidy` function from the `broom` package to summarize the results of the Wilcoxon signed rank test.

```
broom::tidy(wilcox.test(bloodlead$leaddir, conf.int = TRUE,
                       conf.level = 0.90, exact = FALSE))
```

```
estimate statistic p.value conf.low conf.high
1      15.5        499 1.15e-05       11       20.5
method alternative
1 Wilcoxon signed rank test with continuity correction two.sided
```

### 24.5.1 Assumptions of the Wilcoxon Signed Rank procedure

We still must be willing to believe that

1. the paired differences data are a random (or failing that, representative) sample from the population of interest, and
2. that the samples were drawn independently, from an identical population distribution

regardless of what testing procedure we use. But, for the Wilcoxon signed rank test, we also assume

3. that the population distribution of the paired differences is symmetric, but potentially outlier-prone.

### 24.5.2 Calculation Details: The Wilcoxon Signed Rank test

- Calculate the paired difference for each pair, and drop those with difference = 0.
- Let  $N$  be the number of pairs, so there are  $2N$  data points.
- Rank the pairs in order of smallest (rank = 1) to largest (rank =  $N$ ) absolute difference.
- Calculate  $W$ , the sum of the signed ranks by

$$W = \sum_{i=1}^N [sgn(x_{2,i} - x_{1,i})] \prod R_i]$$

- The sign function  $sgn(x) = -1$  if  $x < 0$ , 0 if  $x = 0$ , and  $+1$  if  $x > 0$ .
- Statistical software will convert  $W$  into a  $p$  value, given  $N$ .

Wikipedia's page on the Wilcoxon signed-rank test is a good resource for example calculations.

## 24.6 The Sign test

The **sign test** is something we've skipped in our discussion so far. It is a test for consistent differences between pairs of observations, just as the paired t test, Wilcoxon signed rank test and bootstrap for paired samples can provide. It has the advantage that it is relatively easy to calculate by hand, and that it doesn't require the paired differences to follow a Normal distribution. In fact, it will even work if the data are substantially skewed.

- Calculate the paired difference for each pair, and drop those with difference = 0.
- Let  $N$  be the number of pairs that remain, so there are  $2N$  data points.
- Let  $W$ , the test statistic, be the number of pairs (out of  $N$ ) in which the difference is positive.
- Assuming that  $H_0$  is true, then  $W$  follows a binomial distribution with probability 0.5 on  $N$  trials.

For example, consider our data on blood lead content:

```
bloodlead$leaddir
```

```
[1] 22 5 23 -6 18 25 13 47 15 16 6 1 2 7 0 4 -9 -3 36 25 1 16 42
[24] 30 25 23 32 17 9 -3 60 14 14
```

Difference	# of Pairs
Greater than zero	28
Equal to zero	1
Less than zero	4

So we have  $N = 32$  pairs, with  $W = 28$  that are positive. We can calculate the  $p$  value using the `binom.test` approach in R:

```
binom.test(x = 28, n = 32, p = 0.5, alternative = "two.sided")
```

Exact binomial test

```
data: 28 and 32
number of successes = 30, number of trials = 30, p-value = 2e-05
alternative hypothesis: true probability of success is not equal to 0.5
95 percent confidence interval:
 0.710 0.965
sample estimates:
probability of success
 0.875
```

- A one-tailed test can be obtained by substituting in “less” or “greater” as the alternative of interest.
- The confidence interval provided here doesn't relate back to our original population means, of course. It's just showing the confidence interval around the probability of the exposed mean being greater than the control mean for a pair of children.

## 24.7 Conclusions for the `bloodlead` study

Using any of these procedures, we would conclude that the null hypothesis (that the true mean of the paired differences is 0 mg/dl) is not tenable, and that it should be rejected at the 10% significance level. The smaller the  $p$  value, the stronger is the evidence that the null hypothesis is incorrect, and in this case, we have some fairly tiny  $p$  values.

Procedure	p value	90% CI for $\mu_{Exposed-Control}$	Conclusion
Paired t	$2 \times 10^{-6}$	11.3, 20.6	Reject $H_0$ .
Wilcoxon signed rank	$1 \times 10^{-5}$	11, 20.5	Reject $H_0$ .
Bootstrap CI	$p < 0.10$	11.6, 20.6	Reject $H_0$ .
Sign test	$2 \times 10^{-5}$	None provided	Reject $H_0$ .

Note that **one-sided** or **one-tailed** hypothesis testing procedures work the same way for paired samples as they did for single samples in Section 22.

## 24.8 Building a Decision Support Tool: Comparing Means

1. Are these paired or independent samples?
2. If paired samples, then are the paired differences approximately Normally distributed?
  - a. If yes, then a paired t test or confidence interval is likely the best choice.
  - b. If no, is the main concern outliers (with generally symmetric data), or skew?
    1. If the paired differences appear to be generally symmetric but with substantial outliers, a Wilcoxon signed rank test is an appropriate choice, as is a bootstrap confidence interval for the population mean of the paired differences.
    2. If the paired differences appear to be seriously skewed, then we'll usually build a bootstrap confidence interval, although a sign test is another reasonable possibility.



# Chapter 25

## Comparing Two Means Using Independent Samples

In this section, we apply several methods of testing the null hypothesis that two populations have the same distribution of a quantitative variable, based on independent samples of data. In particular, we'll focus on the comparison of means using independent sample t tests, rank sum tests, and bootstrap approaches. Our example comes from the Ibuprofen in Sepsis trial, which was introduced in Section 18 and then further developed in Section 21

In that trial, 300 patients meeting specific criteria (including elevated temperature) for a diagnosis of sepsis were randomly assigned to either the Ibuprofen group (150 patients) and 150 to the Placebo group. Group information (our exposure) is contained in the `treat` variable. The key outcome of interest to us was `temp_drop`, the change in body temperature (in °C) from baseline to 2 hours later, so that positive numbers indicate drops in temperature (a good outcome.)

```
sepsis %>% select(id, treat, temp_drop)
```

```
# A tibble: 300 x 3
  id      treat temp_drop
  <chr>   <fctr>    <dbl>
1 S002   Ibuprofen     1.4
2 S004   Ibuprofen     0.4
3 S005   Placebo       0.0
4 S006   Ibuprofen    -0.2
5 S009   Ibuprofen     0.6
6 S011   Ibuprofen    -0.4
7 S012   Placebo      -0.1
8 S014   Placebo       0.3
9 S016   Placebo       0.1
10 S020  Ibuprofen     1.5
# ... with 290 more rows
```

### 25.1 Specifying A Two-Sample Study Design

Again, these questions will help specify the details of the study design involved in any comparison of means.

1. What is the outcome under study?
2. What are the (in this case, two) treatment/exposure groups?

3. Were the data collected using matched / paired samples or independent samples?
4. Are the data a random sample from the population(s) of interest? Or is there at least a reasonable argument for generalizing from the sample to the population(s)?
5. What is the significance level (or, the confidence level) we require here?
6. Are we doing one-sided or two-sided testing/confidence interval generation?
7. If we have paired samples, did pairing help reduce nuisance variation?
8. If we have paired samples, what does the distribution of sample paired differences tell us about which inferential procedure to use?
9. If we have independent samples, what does the distribution of each individual sample tell us about which inferential procedure to use?

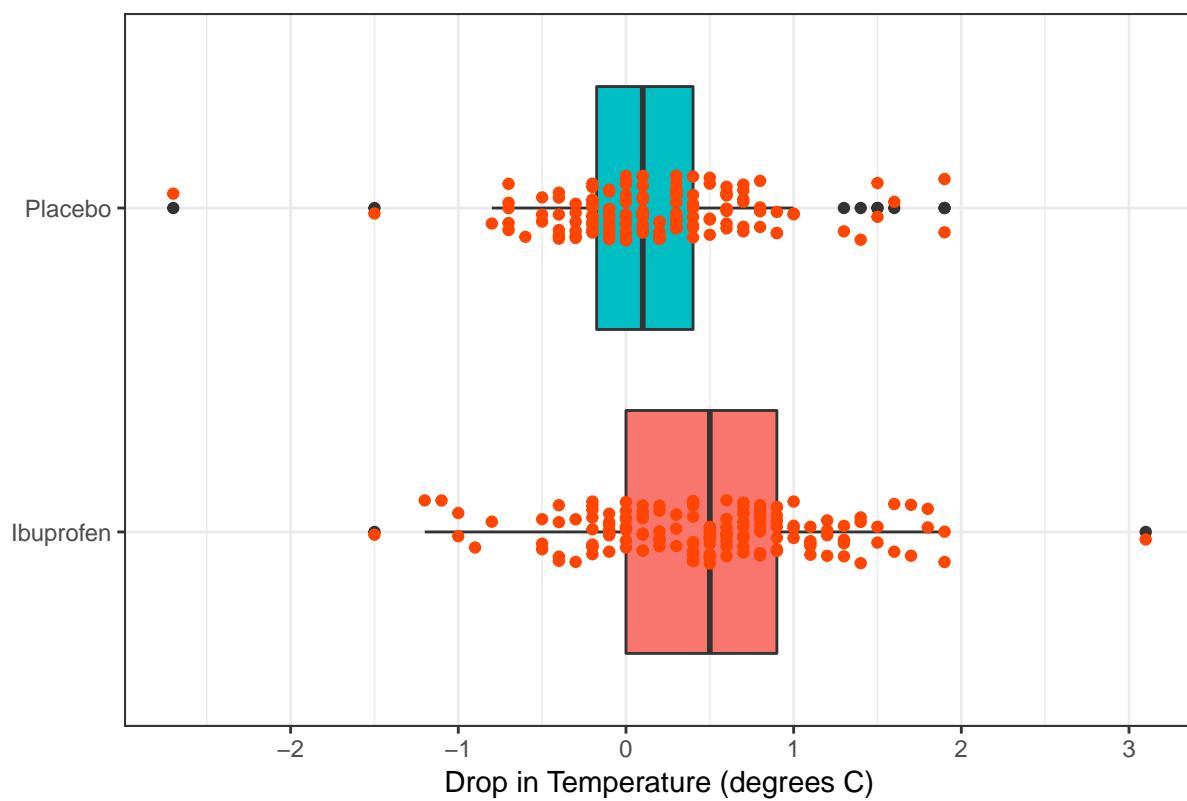
### 25.1.1 For the `sepsis` study

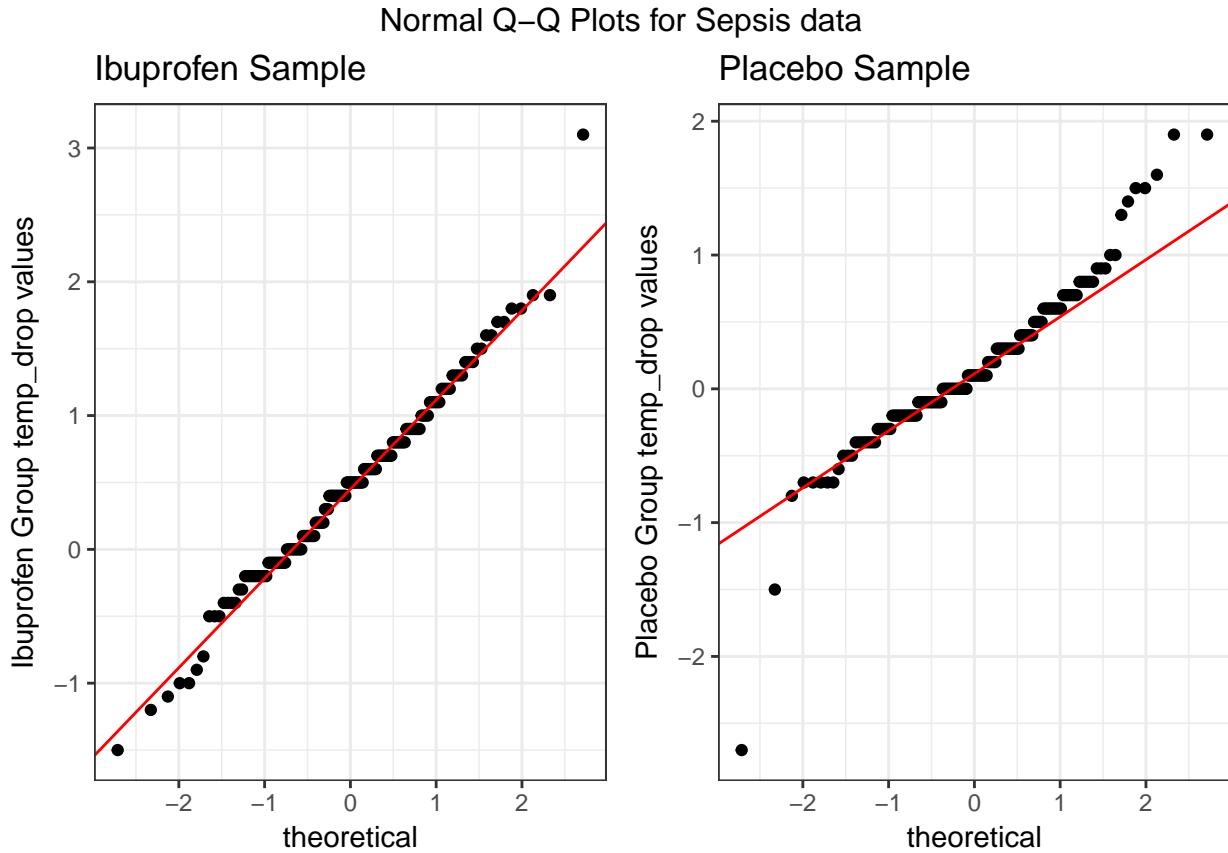
1. The outcome is `temp_drop`, the change in body temperature (in °C) from baseline to 2 hours later, so that positive numbers indicate drops in temperature (a good outcome.)
2. The groups are **Ibuprofen** and **Placebo** as contained in the `treat` variable in the `sepsis` tibble.
3. The data were collected using independent samples. The Ibuprofen subjects are not matched or linked to individual Placebo subjects - they are separate groups.
4. The subjects of the study aren't drawn from a random sample of the population of interest, but they are randomly assigned to their respective treatments (Ibuprofen and Placebo) which will provide the reasoned basis for our inferences.
5. We'll use a 10% significance level (or 90% confidence level) in this setting, as we did in our previous work on these data.
6. We'll use a two-sided testing and confidence interval approach.

Questions 7 and 8 don't apply, because these are independent samples of data, rather than paired samples.

To address question 9, we'll need to look at the data in each sample. We'll repeat the boxplot from Section 18, that allow us to assess the Normality of the distributions of (separately) the `temp_drop` results in the Ibuprofen and Placebo groups.

Boxplot of Temperature Drop in Sepsis Patients





From these plots we conclude that the data in the Ibuprofen sample follow a reasonably Normal distribution, but this isn't as true for the Placebo sample. It's hard to know whether the apparent Placebo group outliers will affect whether the Normal distribution assumption is reasonable, but we'll look into it.

## 25.2 Hypothesis Testing for the Sepsis Example

### 25.2.1 Our Research Question

Is there reasonable evidence, based on these samples of 150 Ibuprofen and 150 Placebo subjects, for us to conclude that those randomly assigned to receive Ibuprofen have a different population mean `temp_drop` than those randomly assigned to receive the Placebo? In other words, if we generated `temp_drop` results for Ibuprofen and Placebo at the population level, would the difference in (say) means be centered at zero, indicating no difference between the two treatments?

### 25.2.2 Specify the null hypothesis

Our null hypothesis here is that the population (true) mean `temp_drop` for subjects receiving Ibuprofen is the same as the population mean `temp_drop` for subjects receiving Placebo plus a constant value (which we'll symbolize with  $\Delta_0$ , which is again usually 0.) Since we have independent samples of data in this trial, we describe this hypothesis in terms of the difference between the separate population means. The hypotheses we are testing are:

- $H_0$ : mean in population 1 = mean in population 2 + hypothesized difference  $\Delta_0$  vs.
- $H_A$ : mean in population 1  $\neq$  mean in population 2 + hypothesized difference  $\Delta_0$ ,

where  $\Delta_0$  is almost always zero. An equivalent way to write this is:

- $H_0 : \mu_1 = \mu_2 + \Delta_0$  vs.
- $H_A : \mu_1 \neq \mu_2 + \Delta_0$

Yet another equally valid way to write this is:

- $H_0 : \mu_1 - \mu_2 = \Delta_0$  vs.
- $H_A : \mu_1 - \mu_2 \neq \Delta_0$ ,

where, again  $\Delta_0$  is almost always zero.

We will generally take this latter approach, where the difference in population means (here, we'll use Ibuprofen - Placebo, but we could have just as easily selected Placebo - Ibuprofen: the order is arbitrary so long as we are consistent) is compared to a constant value, usually 0.

For the `sepsis` example, our population parameters  $\mu_{Ibuprofen}$  and  $\mu_{Placebo}$  are mean temperature drops (in °C) from baseline to 2 hours later, so that positive numbers indicate drops in temperature (a good outcome.)

- Our null hypothesis is that  $\mu_{Ibuprofen} - \mu_{Placebo}$  is 0 degrees.

### 25.2.3 Specify the research hypothesis

The research hypothesis for the sepsis trial is that  $\mu_{Ibuprofen} - \mu_{Placebo}$  is NOT 0 degrees.

### 25.2.4 Specify the test procedure and $\alpha$

As we've seen in Section 21, there are several ways to build a confidence interval to address these hypotheses, and each of those approaches provides information about a related hypothesis test. This includes the pooled t test, the Welch t test, the Wilcoxon-Mann-Whitney rank sum test, and a bootstrap comparison of means (or medians, etc.) using independent samples. We'll specify an  $\alpha$  value of .10 here for the sepsis trial, indicating a 10% significance level (and 90% confidence level.)

### 25.2.5 Calculate the test statistic and $p$ value

Section 21 demonstrated the relevant R code for the `sepsis` example to obtain  $p$  values. For the bootstrap procedure, we again build a confidence interval. We repeat that work below.

### 25.2.6 Draw a conclusion

As we've seen, we use the  $p$  value to either

- **reject**  $H_0$  in favor of the alternative  $H_A$  (concluding that there is a statistically significant difference/association at the  $\alpha$  significance level) if the  $p$  value is less than our desired  $\alpha$  or
- **retain**  $H_0$  (and conclude that there is no statistically significant difference/association at the  $\alpha$  significance level) if the  $p$  value is greater than or equal to  $\alpha$ .

## 25.3 The Pooled T test

The standard method for comparing population means based on two independent samples is based on the t distribution, and requires the following assumptions:

1. [Independence] The samples for the two groups are drawn independently.

2. [Random Samples] The samples for each of the groups are drawn at random from the populations of interest.
3. [Normal Population] The two populations are each Normally distributed
4. [Equal Variances] The population variances in the two groups being compared are the same, so we can obtain a pooled estimate of their joint variance.

### 25.3.1 The Pooled t test Statistic

The test statistic is a t ratio, built up as follows.

$$t_{observed} = \frac{(\bar{x}_1 - \bar{x}_2) - \Delta_0}{SE_{pooled}(\bar{x}_1 - \bar{x}_2)},$$

where  $SE_{pooled}(\bar{x}_1 - \bar{x}_2) = \sqrt{\frac{s^2_{pooled}}{n_1} + \frac{s^2_{pooled}}{n_2}}$  and  $s^2_{pooled} = \frac{(n_1-1)s^2_1 + (n_2-1)s^2_2}{n_1+n_2-2}$  and

where the  $p$  value is found by comparing this observed value  $t_{observed}$  to the t distribution with  $n_1 + n_2 - 2$  degrees of freedom.

### 25.3.2 The Pooled Variances t test in R

The pooled variances t test in R (also called the t test assuming equal population variances) is obtained as follows.

```
t.test(temp_drop ~ treat, data = sepsis, var.equal=TRUE)
```

Two Sample t-test

```
data: temp_drop by treat
t = 4, df = 300, p-value = 3e-05
alternative hypothesis: true difference in means is not equal to 0
95 percent confidence interval:
 0.168 0.455
sample estimates:
mean in group Ibuprofen   mean in group Placebo
          0.464                  0.153
```

We see from the t test output that the test statistic  $t_{observed} = 4.27$  is based on 298 degrees of freedom, which produces a  $p$  value of  $2.7 \times 10^{-5}$ . This  $p$  value is much less than our chosen value for  $\alpha = 0.10$ , so we will clearly **reject** the null hypothesis and conclude that there is a statistically significant difference between the mean temperature drops in the Ibuprofen and Placebo groups.

### 25.3.3 Using broom to tidy the pooled t test

We can use the `tidy` function within the `broom` package to summarize the results of a t test, just as we did with a t-based confidence interval.

```
broom::tidy(t.test(temp_drop ~ treat,
                   data = sepsis, var.equal=TRUE))
```

	estimate1	estimate2	statistic	p.value	parameter	conf.low	conf.high
1	0.464	0.153	4.27	2.68e-05	298	0.168	0.455
	method alternative						
1	Two Sample t-test two.sided						

### 25.3.4 The Pooled T test in a Regression Model

Another way to obtain the pooled t test when comparing two population means using independent samples is to fit a simple regression model, that predicts the outcome of interest using an indicator variable to describe the exposure. For instance, in our `sepsis` trial, we have `temp_drop` as the outcome, and `treat` (Ibuprofen or Placebo) as the predictor in the following model.

```
sepsis.model1 <- lm(temp_drop ~ treat, data = sepsis)
summary(sepsis.model1)
```

```
Call:
lm(formula = temp_drop ~ treat, data = sepsis)

Residuals:
    Min      1Q  Median      3Q     Max 
-2.8527 -0.3640 -0.0527  0.3473  2.6360 

Coefficients:
            Estimate Std. Error t value Pr(>|t|)    
(Intercept)  0.4640    0.0516   8.99 < 2e-16 ***
treatPlacebo -0.3113    0.0730  -4.27  2.7e-05 *** 
---
Signif. codes:  0 '***' 0.001 '**' 0.01 '*' 0.05 '.' 0.1 ' ' 1

Residual standard error: 0.632 on 298 degrees of freedom
Multiple R-squared:  0.0575,    Adjusted R-squared:  0.0544 
F-statistic: 18.2 on 1 and 298 DF,  p-value: 2.68e-05
```

```
confint(sepsis.model1)
```

	2.5 %	97.5 %
(Intercept)	0.362	0.566
treatPlacebo	-0.455	-0.168

The regression model estimates:

- the point estimate for the population mean in the “Ibuprofen” group as 0.464
- the estimated effect of “Placebo” as compared to “Ibuprofen” as  $-0.311$
- the  $p$  value from the pooled t test comparing “Ibuprofen” to “Placebo” as  $2.68 \times 10^{-5}$
- the 95% confidence interval associated with the pooled t test, of  $(-0.455, -0.168)$ , which is just the negative of the result we obtained earlier (in this model, we are estimating Placebo - Ibuprofen, and in our `t.test` output, we estimated Ibuprofen - Placebo)

All of these values, drawn from the regression output above, match the pooled t test results.

## 25.4 The Welch T test

The default confidence interval based on the t test for independent samples in R uses something called the Welch test, in which the two populations being compared are not assumed to have the same variance. Each population is assumed to follow a Normal distribution, though, so the assumptions are:

1. [Independence] The samples for the two groups are drawn independently.
2. [Random Samples] The samples for each of the groups are drawn at random from the populations of interest.
3. [Normal Population] The two populations are each Normally distributed

It turns out that the Welch test gives essentially the same result as the pooled t test when either:

- the design is balanced (our sample contains the same number of subjects in each group), or
- the sample variances (equivalently, standard deviations) are quite similar in the two groups.

In our case, we have a balanced design, and so expect the Welch test and pooled t test to give nearly the same result.

### 25.4.1 The Welch t test in R

The Welch t test in R (also called the t test NOT assuming equal population variances) is obtained as follows.

```
t.test(temp_drop ~ treat, data = sepsis)
```

Welch Two Sample t-test

```
data: temp_drop by treat
t = 4, df = 300, p-value = 3e-05
alternative hypothesis: true difference in means is not equal to 0
95 percent confidence interval:
0.168 0.455
sample estimates:
mean in group Ibuprofen   mean in group Placebo
0.464                      0.153
```

We see from the t test output that the test statistic  $t_{observed} = 4.27$  is based on a fractional degrees of freedom, specifically 288.24 (this fractional df is characteristic of the Welch test) which produces a  $p$  value of  $2.7 \times 10^{-5}$ . Again, since the  $p$  value is less than  $\alpha = 0.10$ , we will **reject** the null hypothesis and conclude that there is a statistically significant difference between the mean temperature drops in the Ibuprofen and Placebo groups.

### 25.4.2 Using broom to tidy the Welch t test

```
broom::tidy(t.test(temp_drop ~ treat,
                   data = sepsis))

estimate estimate1 estimate2 statistic p.value parameter conf.low
1    0.311     0.464     0.153      4.27 2.71e-05      288     0.168
conf.high               method alternative
1    0.455 Welch Two Sample t-test   two.sided
```

## 25.5 Bootstrap CI for $\mu_1 - \mu_2$ from Independent Samples

As we saw in our plots earlier, assuming Normality, particularly in the Placebo population, is hard to justify. So we'll consider methods that don't require that assumption. The bootstrap approach to comparing population means using two independent samples still requires:

1. [Independence] The samples for the two groups are drawn independently.
2. [Random Samples] The samples for each of the groups are drawn at random from the populations of interest.

but does not require either of the other two assumptions.

### 25.5.1 Using the `bootdif` function from `Love-boost.R`

The `bootdif` function contained in the `Love-boost.R` script is a slightly edited version of the function at <http://biostat.mc.vanderbilt.edu/wiki/Main/BootstrapMeansSoftware>. Note that this approach uses a comma to separate the outcome variable (here, `temp_drop`) from the variable identifying the exposure groups (here, `treat`).

As in our previous bootstrap procedures, we are sampling (with replacement) a series of many data sets (default: 2000).

- Here, we are building bootstrap samples based on the SBP levels in the two independent samples (Ibuprofen vs. Placebo).
- For each bootstrap sample, we are calculating a mean difference between the two groups (Ibuprofen vs. Placebo).
- We then determine the 5<sup>th</sup> and 95<sup>th</sup> percentile of the resulting distribution of mean differences (for a 90% confidence interval).

```
set.seed(431212)
bootdif(sepsis$temp_drop, sepsis$treat, conf.level = 0.90)

detach("package:Hmisc", unload=TRUE)
```

Mean Difference	0.05	0.95
-0.3113333	-0.4313333	-0.1973000

Since zero is not contained in this 90% confidence interval, we reject the null hypothesis (that the difference in population means between Ibuprofen and Placebo is zero) at the 10% significance level, so we know that  $p < 0.10$ .

*Note:* Running `bootdif` loads the `Hmisc` package which conflicts with some key functions in the `tidyverse` later. To remove these undesirable effects, I sometimes run `detach("package:Hmisc", unload=TRUE)` as above to try to help.

## 25.6 Wilcoxon-Mann-Whitney Rank Sum Test

The rank sum test is a non-parametric test of whether the two samples were selected from populations having the same distribution. The Wilcoxon-Mann-Whitney Rank Sum test still requires:

1. [Independence] The samples for the two groups are drawn independently.
2. [Random Samples] The samples for each of the groups are drawn at random from the populations of interest.

It also doesn't really compare population means, so in that sense it can be a little confusing.

### 25.6.1 The Test Statistic

- Assign numerical ranks to all observations across the two groups.
  - 1 = smallest, n = largest. Use midpoint for any ties.
- Add up the ranks from sample 1. Call that  $R_1$ .
  - $R_2$  is then known, since the sum of all ranks is  $\frac{n(n+1)}{2}$
  - $U_1 = R_1 - \frac{n_1(n_1+1)}{2}$ , where  $n_1$  is the sample size for sample 1.
  - $U_1 + U_2$  is always just  $n_1 n_2$ , so it doesn't matter which sample you treat as sample 1.
  - The smaller of  $U_1$  and  $U_2$  is then called U, the test statistic.
  - Software converts U into a  $p$  value via a Normal approximation, given  $n_1$  and  $n_2$ .

More details, including an alternative calculation approach, and a worked example are found on the Wikipedia page for the Mann-Whitney U test.

### 25.6.2 Wilcoxon-Mann-Whitney Rank Sum Test in R

```
wilcox.test(temp_drop ~ treat, data = sepsis, conf.int = TRUE)
```

```
Wilcoxon rank sum test with continuity correction

data: temp_drop by treat
W = 10000, p-value = 7e-06
alternative hypothesis: true location shift is not equal to 0
95 percent confidence interval:
 0.2 0.5
sample estimates:
difference in location
                  0.3
```

## 25.7 The Continuity Correction

The  $p$  value for the rank sum test is obtained via a Normal approximation, using the test statistic W.

- That approximation can be slightly improved through the use of a continuity correction (a small adjustment to account for the fact that we're using a continuous distribution, the Normal, to approximate a discretely valued test statistic, W.)
- The continuity correction is particularly important in the case where we have many tied ranks, and is applied by default in R.
- If you want (for some reason) to not use it, add `correct = FALSE` to your call to the `wilcox.test()` function.

### 25.7.1 Tidying a Wilcoxon Rank Sum Test

```
broom::tidy(wilcox.test(temp_drop ~ treat,
                       data = sepsis, conf.int = TRUE))

estimate statistic p.value conf.low conf.high
1       0.3      14614 7.28e-06      0.2       0.5
                                              method alternative
1 Wilcoxon rank sum test with continuity correction two.sided
```

## 25.8 Conclusions for the `sepsis` study

Using any of these procedures, we would conclude that the null hypothesis (that the true difference between the Ibuprofen and Placebo mean temperature drops is 0 degrees) is untenable, and that it should be rejected at the 10% significance level. The smaller the  $p$  value, the stronger is the evidence that the null hypothesis is incorrect, and in this case, we have some fairly tiny  $p$  values.

The sample mean temperature drop for Ibuprofen was 0.464, and the sample mean for Placebo was 0.153.

Procedure	p value	90% CI for $\mu_{Exposed-Control}$	Conclusion
Pooled t test	$2.7 \times 10^{-5}$	0.168, 0.455	Reject $H_0$ .
Welch t test	$2.7 \times 10^{-5}$	0.168, 0.455	Reject $H_0$ .
Wilcoxon-Mann-Whitney rank sum test	$7.3 \times 10^{-6}$	0.2, 0.5	Reject $H_0$ .
Bootstrap CI from bootdif	p < 0.10	0.197, 0.431	Reject $H_0$ .

Note that **one-sided** or **one-tailed** hypothesis testing procedures work the same way for tests as they did for confidence intervals.

## 25.9 A More Complete Decision Support Tool: Comparing Means

1. Are these paired or independent samples?
2. If paired samples, then are the paired differences approximately Normally distributed?
  - a. If yes, then a paired t test or confidence interval is likely the best choice.
  - b. If no, is the main concern outliers (with generally symmetric data), or skew?
    1. If the paired differences appear to be generally symmetric but with substantial outliers, a Wilcoxon signed rank test is an appropriate choice, as is a bootstrap confidence interval for the population mean of the paired differences.
    2. If the paired differences appear to be seriously skewed, then we'll usually build a bootstrap confidence interval, although a sign test is another reasonable possibility.
3. If independent, is each sample Normally distributed?
  - a. No -> use Wilcoxon-Mann-Whitney rank sum test or bootstrap via bootdif.
  - b. Yes -> are sample sizes equal?
    1. Balanced Design (equal sample sizes) - use pooled t test
    2. Unbalanced Design - use Welch test

## 25.10 Paired (Dependent) vs. Independent Samples

One area that consistently trips students up in this course is the thought process involved in distinguishing studies comparing means that should be analyzed using *dependent* (i.e. paired or matched) samples and those which should be analyzed using *independent* samples. A dependent samples analysis uses additional information about the sample to pair/match subjects receiving the various exposures. That additional information is not part of an independent samples analysis (unpaired testing situation.) The reasons to do this are to (a) increase statistical power, and/or (b) reduce the effect of confounding. Here are a few thoughts on the subject.

In the design of experiments, **blocking** is the term often used for the process of arranging subjects into groups (blocks) that are similar to one another. Typically, a blocking factor is a source of variability that is not of primary interest to the researcher. An example of a blocking factor might be the sex of a patient; by blocking on sex, this source of variability is controlled for, thus leading to greater accuracy.

1. If the sample sizes are not balanced (not equal), the samples must be treated as independent, since there would be no way to precisely link all subjects. So, if we have 10 subjects receiving exposure A and 12 subjects receiving exposure B, a dependent samples analysis (such as a paired *t* test) is not correct.
2. The key element is a meaningful link between each observation in one exposure group and a specific observation in the other exposure group. Given a balanced design, the most common strategy indicating dependent samples involves two or more *repeated measures* on the same subjects. For example, if we are comparing outcomes *before* and *after* the application of an exposure, and we have, say, 20 subjects

who provide us data both *before* and *after* the exposure, then the comparison of results *before* and *after* exposure should use a dependent samples analysis. The link between the subjects is the subject itself - each exposed subject serves as its own control.

3. The second most common strategy indicating dependent samples involves deliberate matching of subjects receiving the two exposures. A matched set of observations (often a pair, but it could be a trio or quartet, etc.) is determined using baseline information and then (if a pair is involved) one subject receives exposure A while the other member of the pair receives exposure B, so that by calculating the paired difference, we learn about the effect of the exposure, while controlling for the variables made similar across the two subjects by the matching process.
4. In order for a dependent samples analysis to be used, we need (a) a link between each observation across the exposure groups based on the way the data were collected, *and* (b) a consistent measure (with the same units of measurement) so that paired differences can be calculated and interpreted sensibly.
5. If the samples are collected to facilitate a dependent samples analysis, the correlation of the outcome measurements across the groups will often be moderately strong and positive. If that's the case, then the use of a dependent samples analysis will reduce the effect of baseline differences between the exposure groups, and thus provide a more precise estimate. But even if the correlation is quite small, a dependent samples analysis should provide a more powerful estimate of the impact of the exposure on the outcome than would an independent samples analysis with the same number of observations.

### 25.10.1 Three “Tricky” Examples

1. Suppose we take a convenient sample of 200 patients from the population of patients who complete a blood test in April 2017 including a check of triglycerides, and who have a triglyceride level in the high category (200 to 499 mg/dl). Next, we select a patient at random from this group of 200 patients, and then identify another patient from the group of 200 who is the same age (to within 2 years) and also the same sex. We then randomly assign our intervention to one of these two patients and usual care without our intervention to the other patient. We then set these two patients aside and return to our original sample, repeating the process until we cannot find any more patients in the same age range and of the same gender. This generates a total of 77 patients who receive the intervention and 77 who do not. If we are trying to assess the effect of our intervention on triglyceride level in October 2017 using this sample of 154 people, should we use dependent (paired) or independent samples?
2. Suppose we take a convenient sample of 77 patients from the population of patients who complete a blood test in April 2017 including a check of triglycerides, and who have a triglyceride level in the high category (200 to 499 mg/dl). Next, we take a convenient sample of 77 patients from the population of patients who complete a blood test in May 2017 including a check of triglycerides, and who have a triglyceride level in the high category (200 to 499 mg/dl). We flip a coin to determine whether the intervention will be given to each of the 77 patients from April 2017 (if the coin comes up “HEADS”) or instead to each of the 77 patients from May 2017 (if the coin comes up “TAILS”). Then, we assign our intervention to the patients seen in the month specified by the coin and assign usual care without our intervention to the patients seen in the other month. If we are trying to assess the effect of our intervention on triglyceride level in October 2017 using this sample of 154 people, should we use dependent (paired) or independent samples?
3. Suppose we take a convenient sample of 200 patients from the population of patients who complete a blood test in April 2017 including a check of triglycerides, and who have a triglyceride level in the high category (200 to 499 mg/dl). For each patient, we re-measure them again in October 2017, again checking their triglyceride level. But in between, we take the first 77 of the patients in a randomly sorted list and assign them to our intervention (which takes place from June through September 2017) and take an additional group of 77 patients from the remaining part of the list and assign them to usual care without our intervention over the same time period. If we are trying to assess the effect of our intervention on each individual’s change in triglyceride level (from April/May to October) using this sample of 154 people, should we use dependent (paired) or independent samples?

### 25.10.2 Answers for the Three “Tricky” Examples

*Answer for 1.* Our first task is to identify the outcome and the exposure groups. Here, we are comparing the distribution of our outcome (triglyceride level in October) across two exposures: (a) receiving the intervention and (b) not receiving the intervention. We have a sample of 77 patients receiving the intervention, and a different sample of 77 patients receiving usual care. Each of the 77 subjects receiving the intervention is matched (on age and sex) to a specific subject not receiving the intervention. So, we can calculate paired differences by taking the triglyceride level for the exposed member of each pair and subtracting the triglyceride level for the usual care member of that same pair. Thus our comparison of the exposure groups should be accomplished using a *dependent* samples analysis, such as a paired t test.

*Answer for 2.* Again, we begin by identifying the outcome (triglyceride level in October) and the exposure groups. Here, we compare two exposures: (a) receiving the intervention and (b) receiving usual care. We have a sample of 77 patients receiving the intervention, and a different sample of 77 patients receiving usual care. But there is no pairing or matching involved. There is no connection implied by the way that the data were collected that implies that, for example, patient 1 in the intervention group is linked to any particular subject in the usual care group. So we need to analyze the data using independent samples.

*Answer for 3.* Once again, we identify the outcome (now it is the within-subject *change* in triglyceride level from April to October) and the exposure groups. Here again, we compare two exposures: (a) receiving the intervention and (b) receiving usual care. We have a sample of 77 patients receiving the intervention, and a different sample of 77 patients receiving usual care. But again, there is no pairing or matching between the patients receiving the intervention and the patients receiving usual care. While each outcome value is a difference (or change) in triglyceride levels, there's no connection implied by the way that the data were collected that implies that, for example, patient 1 in the intervention group is linked to any particular subject in the usual care group. So, again, we need to analyze the data using independent samples.

For more background and fundamental material, you might consider the Wikipedia pages on Paired Difference Test and on Blocking (statistics).



# Chapter 26

## Power and Sample Size Issues Comparing Two Means

### 26.1 Paired Sample t Tests and Power/Sample Size

For a paired-samples t test, R can estimate any one of the following elements, given the other four, using the `power.t.test` command, for either a one-tailed or two-tailed paired t test...

- $n$  = the sample size (# of pairs) being compared
- $\delta$  = delta = the true difference in means between the two groups
- $s$  =  $sd$  = the true standard deviation of the paired differences
- $\alpha$  = `sig.level` = the significance level for the comparison (maximum acceptable risk of Type I error)
- $1 - \beta$  = power = the power of the paired t test to detect the effect of size  $\delta$

### 26.2 A Toy Example

As a toy example, suppose you are planning a paired samples experiment involving  $n = 30$  subjects who will each provide a “Before” and an “After” result, which is measured in days.

Suppose you want to do a two-sided (two-tailed) test at 95% confidence (so  $\alpha = 0.05$ ), and that you expect that the true difference between the “Before” and “After” groups will have to be at least  $\delta = 5$  days to be of any real interest. Suppose also that you are willing to assume that the true standard deviation of those paired differences will be 10 days.

That is, of course, a lot to suppose.

Now, we want to know what power the proposed experiment will have to detect this difference with these specifications, and how tweaking these specifications will affect the power of the study.

So, we have -  $n = 30$  paired differences will be collected -  $\delta = 5$  days is the minimum clinically meaningful difference -  $s = 10$  days is the assumed population standard deviation of the paired differences -  $\alpha$  is 0.05, and we'll do a two-sided test

### 26.3 Using the `power.t.test` function

```
power.t.test(n = 30, delta = 5, sd = 10, sig.level = 0.05,
             type="paired", alternative="two.sided")
```

Paired t test power calculation

```
n = 30
delta = 5
sd = 10
sig.level = 0.05
power = 0.754
alternative = two.sided
```

NOTE: n is number of \*pairs\*, sd is std.dev. of \*differences\* within pairs

So, under this study design, we would expect to detect an effect of size  $\delta = 5$  days with 75% power, i.e. with a probability of incorrect retention of  $H_0$  of 0.25. Most of the time, we'd like to improve this power, and to do so, we'd need to adjust our assumptions.

## 26.4 Changing Assumptions in a Power Calculation

We made assumptions about the sample size n, the minimum clinically meaningful difference in means  $\delta$ , the population standard deviation s, and the significance level  $\alpha$ , not to mention decisions about the test, like that we'd do a paired t test, rather than another sort of test for paired samples, or use an independent samples approach, and that we'd do a two-tailed, or two-sided test. Often, these assumptions are tweaked a bit to make the power look more like what a reviewer/funder is hoping to see.

### 26.4.1 Increasing the Sample Size, absent other changes, will Increase the Power

Suppose, we committed to using more resources and gathering “Before” and “After” data from 40 subjects instead of the 30 we assumed initially – what effect would this have on our power?

```
power.t.test(n = 40, delta = 5, sd = 10, sig.level = 0.05,
             type="paired", alternative="two.sided")
```

Paired t test power calculation

```
n = 40
delta = 5
sd = 10
sig.level = 0.05
power = 0.869
alternative = two.sided
```

NOTE: n is number of \*pairs\*, sd is std.dev. of \*differences\* within pairs

With more samples, we should have a more powerful test, able to detect the difference with greater probability. In fact, a sample of 40 paired differences yields 87% power. As it turns out, we would need at least 44 paired differences with this scenario to get to 90% power, as shown in the calculation below, which puts the power in, but leaves out the sample size.

```
power.t.test(power=0.9, delta = 5, sd = 10, sig.level = 0.05,
            type="paired", alternative="two.sided")
```

Paired t test power calculation

```
n = 44
delta = 5
sd = 10
sig.level = 0.05
power = 0.9
alternative = two.sided
```

NOTE: n is number of \*pairs\*, sd is std.dev. of \*differences\* within pairs

We see that we would need at least 44 paired differences to achieve 90% power. Note: we always round the sample size up in doing a power calculation – if this calculation had actually suggested  $n = 43.1$  paired differences were needed, we would still have rounded up to 44.

### 26.4.2 Increasing the Effect Size, absent other changes, will increase the Power

A larger effect should be easier to detect. If we go back to our original calculation, which had 75% power to detect an effect (i.e. a true population mean difference) of size  $\delta = 5$ , and now change the desired effect size to  $\delta = 6$ , we should obtain a more powerful design.

```
power.t.test(n = 30, delta = 6, sd = 10, sig.level = 0.05,
              type="paired", alternative="two.sided")
```

Paired t test power calculation

```
n = 30
delta = 6
sd = 10
sig.level = 0.05
power = 0.888
alternative = two.sided
```

NOTE: n is number of \*pairs\*, sd is std.dev. of \*differences\* within pairs

We see that this change in effect size from 5 to 6, leaving everything else the same, increases our power from 75% to nearly 89%. To reach 90% power, we'd need to increase the effect size we were trying to detect to at least 6.13 days.

- Again, note that I am rounding up here.
- Using  $\delta = 6.12$  would not quite make it to 90.00% power.
- Using  $\delta=6.13$  guarantees that the power will be 90% or more, and not just round up to 90%..

### 26.4.3 Decreasing the Standard Deviation, absent other changes, will increase the Power

The choice of standard deviation is usually motivated by a pilot study, or else pulled out of thin air. It's relatively easy to convince yourself that the true standard deviation might be a little smaller than you'd guessed initially. Let's see what happens to the power if we reduce the sample standard deviation from

10 days to 9 days. This should make the effect of 5 days easier to detect as being different from the null hypothesized value of 0, because it will have smaller variation associated with it.

```
power.t.test(n = 30, delta = 5, sig.level = 0.05,
             type="paired", alternative="two.sided")
```

Paired t test power calculation

```
n = 30
delta = 5
sd = 9
sig.level = 0.05
power = 0.837
alternative = two.sided
```

NOTE: n is number of \*pairs\*, sd is std.dev. of \*differences\* within pairs

This change in standard deviation from 10 to 9, leaving everything else the same, increases our power from 75% to nearly 84%. To reach 90% power, we'd need to decrease the standard deviation of the population paired differences to no more than 8.16 days.

Note I am rounding down here, because using  $s = 8.17$  days would not quite make it to 90.00% power. Note also that in order to get R to treat the sd as unknown, I must specify it as NULL in the formula...

```
power.t.test(n = 30, delta = 5, sd = NULL, power = 0.9,
             sig.level = 0.05, type="paired", alternative="two.sided")
```

Paired t test power calculation

```
n = 30
delta = 5
sd = 8.16
sig.level = 0.05
power = 0.9
alternative = two.sided
```

NOTE: n is number of \*pairs\*, sd is std.dev. of \*differences\* within pairs

#### 26.4.4 Tolerating a Larger $\alpha$ (Significance Level), without other changes, increases Power

We can trade off some of our Type II error (lack of power) for Type I error. If we are willing to trade off some Type I error (as described by the  $\alpha$ ), we can improve the power. For instance, suppose we decided to run the original test with 90% confidence.

```
power.t.test(n = 30, delta = 5, sd = 10, sig.level = 0.1,
             type="paired", alternative="two.sided")
```

Paired t test power calculation

```
n = 30
delta = 5
sd = 10
```

```

sig.level = 0.1
power = 0.848
alternative = two.sided

```

NOTE: n is number of \*pairs\*, sd is std.dev. of \*differences\* within pairs

The calculation suggests that our power would thus increase from 75% to nearly 85%.

## 26.5 Two Independent Samples: Power for t Tests

For an independent-samples t test, with a balanced design (so that  $n_1 = n_2$ ), R can estimate any one of the following elements, given the other four, using the `power.t.test` command, for either a one-tailed or two-tailed t test...

- n = the sample size in each of the two groups being compared
- $\delta$  = delta = the true difference in means between the two groups
- s = sd = the true standard deviation of the individual values in each group (assumed to be constant – since we assume equal population variances)
- $\alpha$  = sig.level = the significance level for the comparison (maximum acceptable risk of Type I error)
- $1 - \beta$  = power = the power of the t test to detect the effect of size  $\delta$

This method only produces power calculations for balanced designs – where the sample size is equal in the two groups. If you want a two-sample power calculation for an unbalanced design, you will need to use a different library and function in R, as we'll see.

## 26.6 A New Example

Suppose we plan a study of the time to relapse for patients in a drug trial, where subjects will be assigned randomly to a (new) treatment or to a placebo. Suppose we anticipate that the placebo group will have a mean of about 9 months, and want to detect an improvement (increase) in time to relapse of 50%, so that the treatment group would have a mean of at least 13.5 months. We'll use  $\alpha = .10$  and  $\beta = .10$ , as well. Assume we'd do a two-sided test, with an equal number of observations in each group, and we'll assume the observed standard deviation of 9 months in a pilot study will hold here, as well.

We want the sample size required by the test under a two sample setting where:

- $\alpha = .10$ ,
- with 90% power (so that  $\beta = .10$ ),
- and where we will have equal numbers of samples in the placebo group (group 1) and the treatment group (group 2).
- We'll plug in the observed standard deviation of 9 months.
- We'll look at detecting a change from 9 [the average in the placebo group] to 13.5 (a difference of 50%, giving delta = 4.5)
- using a two-sided pooled t-test.

The appropriate R command is:

```

power.t.test(delta = 4.5, sd = 9,
             sig.level = 0.10, power = 0.9,
             type="two.sample",
             alternative="two.sided")

```

Two-sample t test power calculation

```
n = 69.2
delta = 4.5
sd = 9
sig.level = 0.1
power = 0.9
alternative = two.sided
```

NOTE: n is number in \*each\* group

This suggests that we will need a sample of at least 70 subjects in the treated group and an additional 70 subjects in the placebo group, for a total of 140 subjects.

### 26.6.1 Another Scenario

What if resources are sparse, and we'll be forced to do the study with no more than 120 subjects, overall? If we require 90% confidence in a two-sided test, what power will we have?

```
power.t.test(n = 60, delta = 4.5, sd = 9,
             sig.level = 0.10,
             type="two.sample",
             alternative="two.sided")
```

Two-sample t test power calculation

```
n = 60
delta = 4.5
sd = 9
sig.level = 0.1
power = 0.859
alternative = two.sided
```

NOTE: n is number in \*each\* group

It looks like the power under those circumstances would be just under 86%. Note that the n = 60 refers to half of the total sample size, since we'll need 60 drug and 60 placebo subjects in this balanced design.

## 26.7 Power for Independent Sample T tests with Unbalanced Designs

Using the `pwr` library, R can do sample size calculations that describe the power of a two-sample t test that does not require a balanced design using the `pwr.t2n.test` command.

Suppose we wanted to do the same study as we described above, using 100 “treated” patients but as few “placebo” patients as possible. What sample size would be required to maintain 90% power? There is one change here – the effect size d in the `pwr.t2n.test` command is specified using the difference in means  $\delta$  that we used previously, divided by the standard deviation s that we used previously. So, in our old setup, we assumed delta = 4.5, sd = 9, so now we'll assume d = 4.5/9 instead.

```
pwr::pwr.t2n.test(n1 = 100, d = 4.5/9,
                   sig.level = 0.1, power = 0.9,
                   alternative="two.sided")
```

```
t test power calculation

n1 = 100
n2 = 52.8
d = 0.5
sig.level = 0.1
power = 0.9
alternative = two.sided
```

We would need at least 53 subjects in the “placebo” group.

### 26.7.1 The most efficient design for an independent samples comparison will be balanced.

- Note that if we use  $n_1 = 100$  subjects in the treated group, we need at least  $n_2 = 53$  in the placebo group to achieve 90% power, and a total of 153 subjects.
- Compare this to the balanced design, where we needed 70 subjects in each group to achieve the same power, thus, a total of 140 subjects.

We saw earlier that a test with 60 subjects in each group would yield just under 86% power. Suppose we instead built a test with 80 subjects in the treated group, and 40 in the placebo group, then what would our power be?

```
pwr::pwr.t2n.test(n1 = 80, n2 = 40, d = 4.5/9,
                   sig.level = 0.10,
                   alternative="two.sided")
```

```
t test power calculation

n1 = 80
n2 = 40
d = 0.5
sig.level = 0.1
power = 0.822
alternative = two.sided
```

As we'd expect, the power is stronger for a balanced design than for an unbalanced design with the same overall sample size.

Note that I used a two-sided test to establish my power calculation – in general, this is the most conservative and defensible approach for any such calculation, **unless there is a strong and specific reason to use a one-sided approach in building a power calculation, don't.**



# Chapter 27

## A Review: Two Examples, Comparing Means

### 27.1 A Study of Battery Life

Should you buy generic rather than brand-name batteries? Bock, Velleman, and De Veaux (2004) describe a designed experiment to test battery life. A (male) student obtained six pairs of AA alkaline batteries from two major battery manufacturers; a well-known brand name and a generic brand, so that battery brand was the factor of interest.

To estimate the difference in mean lifetimes across the two manufacturers, the student kept a battery-powered CD player with the same CD running continuously, with the volume control fixed at 5, and measured the time until no more music was heard through the headphones. (He ran an initial trial to find out approximately how long that would take, so he didn't have to spend the first 3 hours of each run listening to the same CD.) The outcome was the time in minutes until the sound stopped. To account for changes in the CD player's performance over time, he randomized the run order by choosing pairs of batteries (the CD-player required two batteries to run) at random.

Here are the results for the 6 brand name and 6 generic tests, in minutes, found in the `battery.csv` data file, where `run` indicates the order in which the tests were run...

```
battery
```

```
# A tibble: 12 x 4
  run  test      type   time
  <int> <int>    <fctr> <dbl>
1     1     1 brand name   191
2     2     2 brand name   206
3     6     3 brand name   199
4     8     4 brand name   172
5     9     5 brand name   184
6    12     6 brand name   170
7     3     1 generic    194
8     4     2 generic    204
9     5     3 generic    204
10    7     4 generic    206
11   10     5 generic    222
12   11     6 generic    209
```

### 27.1.1 Question 1. What is the outcome under study?

We are studying battery lifetimes (time until the sound stopped) in minutes.

### 27.1.2 Question 2. What are the treatment/exposure groups?

We are comparing the two brands of batteries: the well-known vs. the generic.

### 27.1.3 Question 3. Are the data collected using paired or independent samples?

Of course, if we had different numbers of samples in the two groups, then we'd know without further thought that independent samples were required. Since we have 6 observations in the brand name group, and also have 6 observations in the generic group, i.e. a balanced design, we need to pause now to decide whether paired or independent samples testing is appropriate in this setting.

Two samples are paired if each data point in one sample is naturally linked to a specific data point in the other sample. So, do we have paired or independent samples?

- Despite the way I've set up the data table, there is no particular reason to pair, say, run #1 (a brand name run) with any particular experimental run in the generic group. So the samples are independent. This is not a matched-pairs design.
- In each trial, the student either used two of the well-known batteries, or two of the generic batteries.
- Any of the tests/confidence intervals for the independent samples methods suggests a statistically significant (at the 5% level) difference between the generic and brand name batteries.

### 27.1.4 Question 4. Are the data a random sample from the population of interest?

Probably not. The data are likely to come from a convenient sample of batteries. I don't know how this might bias the study, though. It seems unlikely that there would be a particular bias unless, for example, the well-known batteries were substantially older or younger than the generic.

### 27.1.5 Question 5. What significance level will we use?

We have no reason not to use a 95% confidence level, so  $\alpha = 0.05$

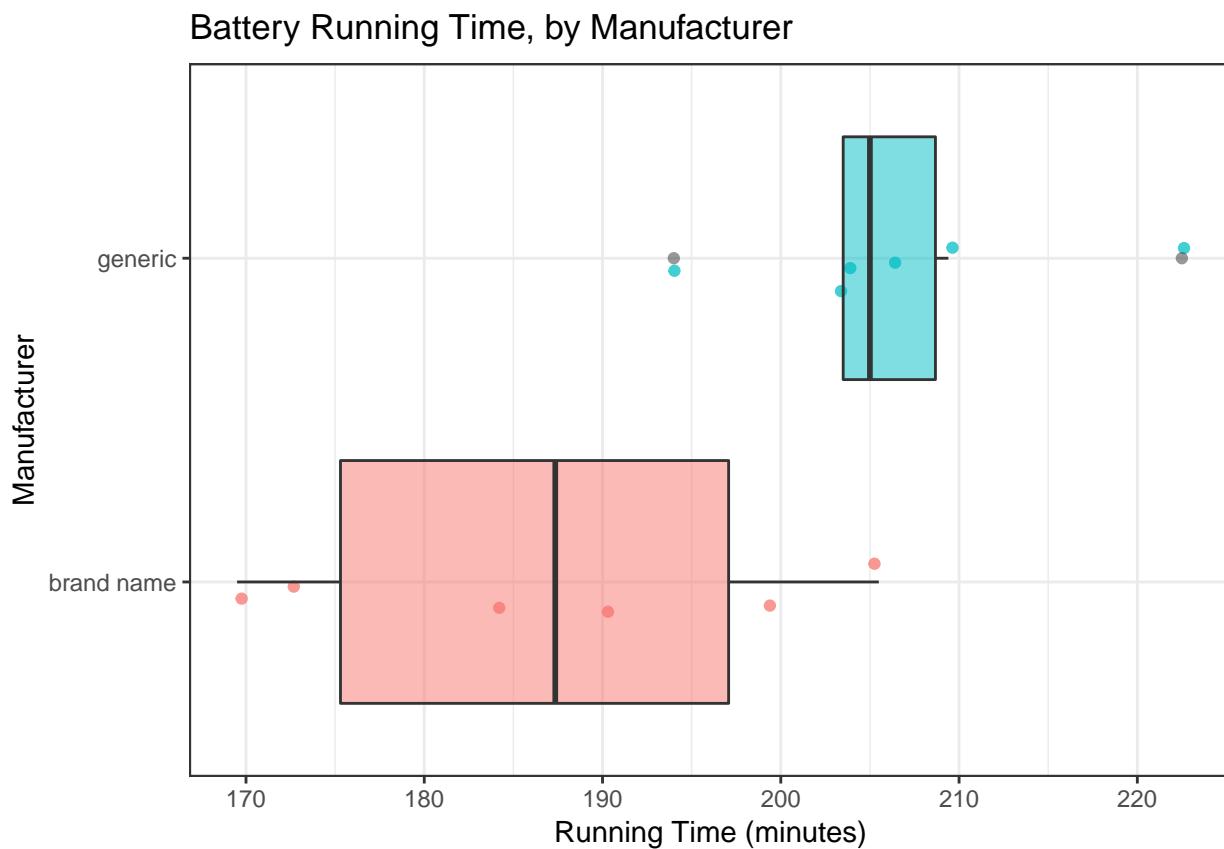
### 27.1.6 Question 6. Are we using a one-sided or two-sided comparison?

We could argue for a one-sided comparison, but I'll be safe and use the two-sided version.

### 27.1.7 Question 9. What does the distribution of outcomes in each group tell us?

```
ggplot(battery, aes(x = type, y = time, fill = type)) +
  geom_jitter(aes(color = type), alpha = 0.75, width = 0.125) +
  geom_boxplot(alpha = 0.5) +
  theme_bw() +
  coord_flip() +
  guides(fill = FALSE, col = FALSE) +
```

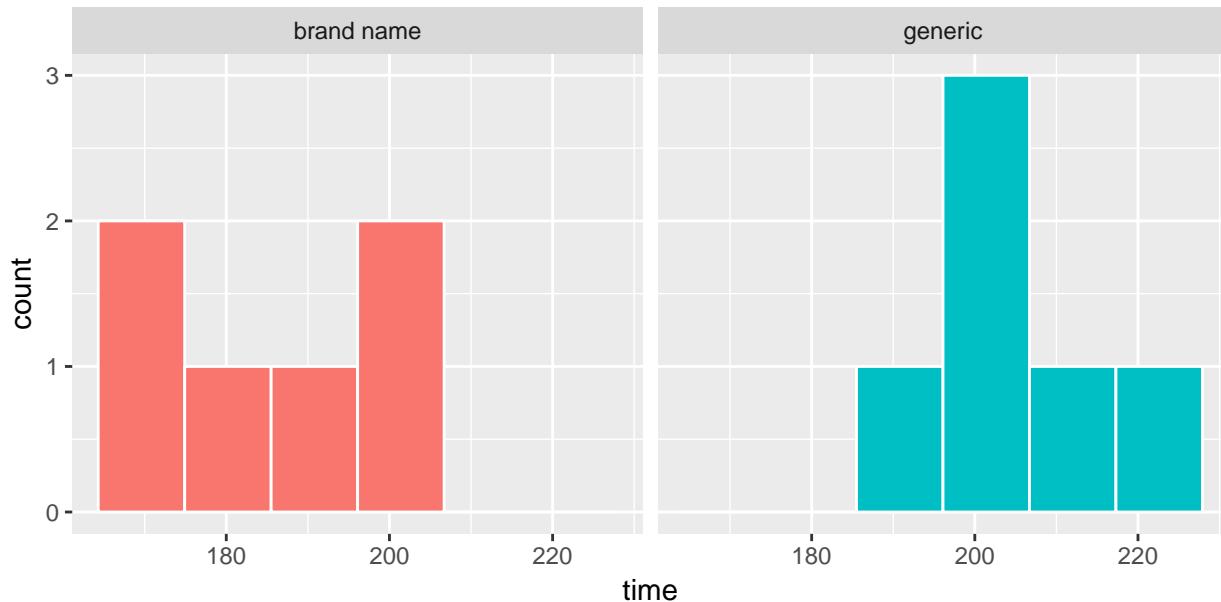
```
labs(title = "Battery Running Time, by Manufacturer",
     y = "Running Time (minutes)", x = "Manufacturer")
```



We can generate histograms, too, but that's an issue, because we have so few observations.

```
ggplot(battery, aes(x = time, fill = type)) +
  geom_histogram(bins = 6, col = "white") +
  facet_wrap(~ type) +
  guides(fill = FALSE) +
  labs(title = "Battery Running Time, by Manufacturer")
```

### Battery Running Time, by Manufacturer



```
by(battery$time, battery$type, mosaic::favstats)
```

```
battery$type: brand name
  min   Q1 median   Q3 max mean   sd n missing
 170  175    187  197  206  187 14.4  6      0
-----
battery$type: generic
  min   Q1 median   Q3 max mean   sd n missing
 194  204    205  209  222  207 9.37  6      0
```

It sure looks like the generic batteries lasted longer. And they also look like they were more consistent. The sample means are 206.6 for the generic group, 186.9 minutes for brand name, so the point estimate of the difference is 19.7 minutes.

The question is: can we be confident that the difference we observe here is more than just random fluctuation, at a 5% significance level?

#### 27.1.8 Inferential Results for the Battery Study

In the table below, I have summarized the two-sided testing results for most of the ways in which we have looked at a two sample comparison so far, with 95% confidence intervals. If the samples really are paired, then we must choose from the paired samples comparisons described in the table. If the samples really are independent, then we must choose from the independent samples comparisons.

#### 27.1.9 Paired Samples Approaches

Method	p Value	95% CI for Generic - Brand Name
Paired t	0.058	-1.0, 40.4
Wilcoxon signed rank	0.063	-2.0, 39.9
Bootstrap via smean.cl.boot	-	6.7, 33.0

### 27.1.10 Independent Samples Approaches

	Method	p Value	95% CI for Generic - Brand Name
	Pooled t	0.018	4.1, 35.3
	Welch's t	0.021	3.7, 35.6
	Wilcoxon Mann Whitney rank sum	0.030	3.3, 37.0
	Bootstrap via boottdif	-	7.7, 32.2

## 27.2 The Breakfast Study: Does Oat Bran Cereal Lower Serum LDL Cholesterol?

Norman and Streiner (2014) describe a crossover study that was conducted to investigate whether oat bran cereal helps to lower serum cholesterol levels in hypercholesterolemic males. Fourteen such individuals were randomly placed on a diet that included either oat bran or corn flakes; after two weeks, their low-density lipoprotein (LDL) cholesterol levels, in mmol/l were recorded. Each subject was then switched to the alternative diet. After a second two-week period, the LDL cholesterol level of each subject was again recorded.

`breakfast`

```
# A tibble: 14 x 3
  subject cornflakes oatbran
    <int>     <dbl>    <dbl>
1       1      4.61    3.84
2       2      6.42    5.57
3       3      5.40    5.85
4       4      4.54    4.80
5       5      3.98    3.68
6       6      3.82    2.96
7       7      5.01    4.41
8       8      4.34    3.72
9       9      3.80    3.49
10      10     4.56    3.84
11      11     5.35    5.26
12      12     3.89    3.73
13      13     2.25    1.84
14      14     4.24    4.14
```

### 27.2.1 Question 1. What is the outcome under study?

We are studying levels of LDL cholesterol, in mmol/l. Note that if we wanted to convert to a more familiar scale, specifically mg/dl, we would multiply the mmol/l by 18, as it turns out.

### 27.2.2 Question 2. What are the treatment/exposure groups?

We are comparing subjects after two weeks of eating corn flakes to the same subjects after two weeks of eating oat bran.

### 27.2.3 Question 3. Are the data collected using paired or independent samples?

These are matched pairs, paired by subject. Each subject produced an oat bran result and a corn flakes result.

### 27.2.4 Question 4. Are the data a random sample from the population of interest?

Probably not. The data are likely to come from a convenient sample of 14 individuals but they were randomly assigned to cornflakes first or to oat bran first, then crossed over.

### 27.2.5 Question 5. What significance level will we use?

We have no reason not to use our usual 95% confidence level, so  $\alpha = 0.05$

### 27.2.6 Question 6. Are we using a one-sided or two-sided comparison?

We could argue for a one-sided comparison, but I'll be safe and use the two-sided version.

### 27.2.7 Question 7. Did pairing help reduce nuisance variation?

After we drop the `breakfast.csv` file into the `breakfast` data frame, we look at the correlation of cornflakes and oatbran results across our 14 subjects.

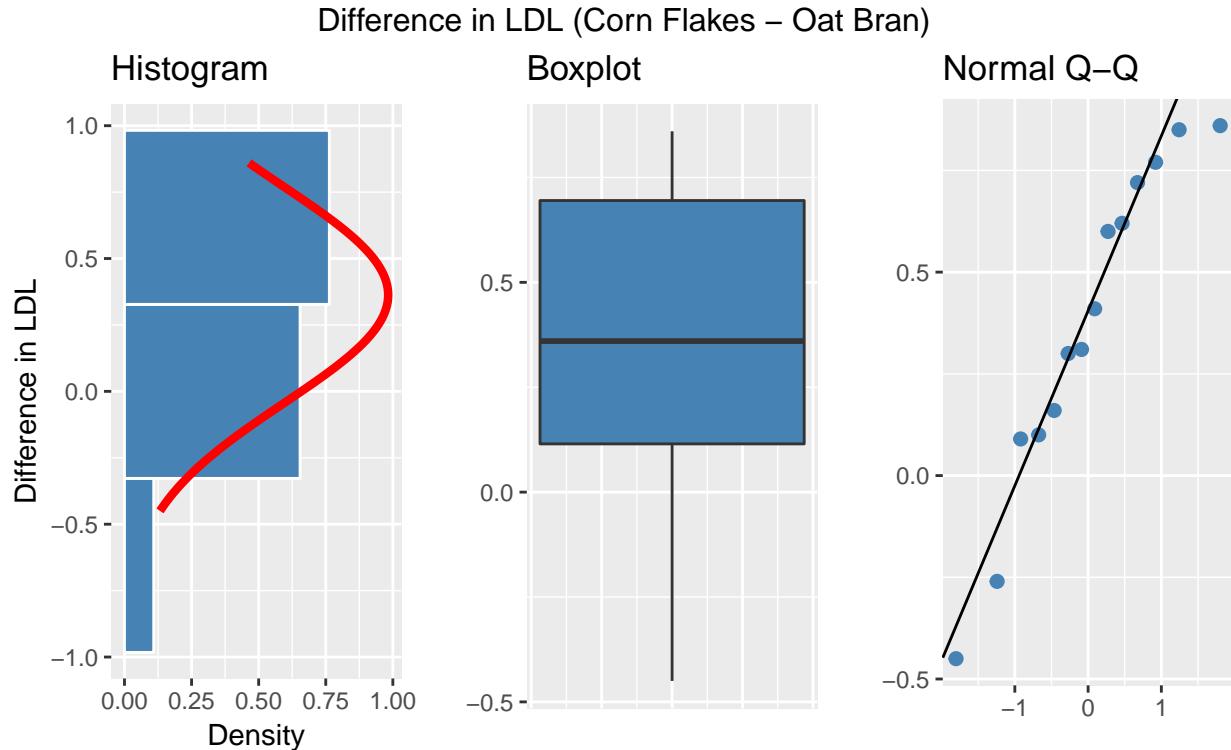
```
cor(breakfast$cornflakes, breakfast$oatbran)
```

```
[1] 0.923
```

The sample Pearson correlation coefficient is very strong and positive at 0.92, so the paired samples approach will use these data far more effectively than the (incorrect) independent samples approach.

### 27.2.8 Question 8. What does the distribution of paired differences tell us?

We summarize the distribution of the paired differences (cornflakes - oatbran) below.



The Normal distribution doesn't look too ridiculous in this case for the paired (cornflakes-oatbran) differences. Suppose we assume Normality and run the paired t test.

```
t.test(breakfast$cornflakes - breakfast$oatbran)
```

#### One Sample t-test

```
data: breakfast$cornflakes - breakfast$oatbran
t = 3, df = 10, p-value = 0.005
alternative hypothesis: true mean is not equal to 0
95 percent confidence interval:
 0.128 0.597
sample estimates:
mean of x
 0.363
```

Based on this sample of 14 subjects in the crossover study, we reject  $H_0$  here and conclude that there is a statistically significant (detectable) difference between the LDL cholesterol levels after eating corn flakes and eating oat bran, at a 5% significance level.

## 27.3 Power, Sample Size and the Breakfast Study

These results look very promising. Suppose that in a new study, you wish to be able to detect a difference in LDL cholesterol between two exposures: subjects who eat cornflakes (as in the original study) and subjects who continue to eat cornflakes but also take a supplemental dosage of what you believe to be the crucial ingredient in oatbran.

Suppose you believe that the effect of taking the new supplement will be about half the size of the effect

you observed in the original breakfast study on hypercholesterolemic males, but that males generally may be more likely to take your supplement regularly than switch from cornflakes to a less appetizing breakfast choice, making your supplement attractive.

What sample size will be required to yield 90% power to detect an effect half the size of the effect we observed in the breakfast study, in a new paired samples study using a two-tailed 5% significance level? What if we only required 80% power?

### 27.3.1 The Setup

We want to know  $n$ , the minimum required sample size for the new study, and we have:

- A specified effect size of half of what we saw in the breakfast study, where the sample mean difference between cornflakes and oatbran was 0.36 mmol/l, so our effect size is assumed to be  $\delta = 0.18$  mmol/l.
- An assumed standard deviation equal to the standard deviation of the differences in the pilot breakfast study, which turns out to have been  $s = 0.41$  mmol/l.
- We also have a pre-specified  $\alpha = 0.05$  using a two-tailed test.
- We also want the power to be at least 90% for our new study.

### 27.3.2 The R Calculations

**Question 1.** What sample size will be required to yield 90% power to detect an effect half the size of the effect we observed in the breakfast study, in a new paired samples study using a two-tailed 5% significance level?

```
power.t.test(delta = 0.18, sd = 0.41, sig.level = 0.05,
             power = 0.9, type="paired", alternative="two.sided")
```

Paired t test power calculation

```
n = 56.5
delta = 0.18
sd = 0.41
sig.level = 0.05
power = 0.9
alternative = two.sided
```

NOTE: n is number of \*pairs\*, sd is std.dev. of \*differences\* within pairs

And so our new study will require at least **57 subjects** (each measured in two circumstances, so 114 total measurements) in order to achieve at least 90% power to detect the difference of 0.18 mmol/l while meeting these specifications.

**Question 2.** What if we were willing to accept only 80% power?

```
power.t.test(delta = 0.18, sd = 0.41, sig.level = 0.05,
             power = 0.8, type="paired", alternative="two.sided")
```

Paired t test power calculation

```
n = 42.7
delta = 0.18
sd = 0.41
sig.level = 0.05
```

```
power = 0.8
alternative = two.sided
```

NOTE: n is number of \*pairs\*, sd is std.dev. of \*differences\* within pairs

It turns out that this would require at least **43 subjects**.

### 27.3.3 Independent samples, instead of paired samples?

What would happen if, instead of doing a paired samples study, we did one using independent samples? Assuming we used a balanced design, and assigned the same number of different people at random to either the oatbran supplement or regular cornflakes alone, we could do such a study, but it would require many more people to obtain similar power to the paired samples study.

```
power.t.test(delta = 0.18, sd = 0.41, sig.level = 0.05,
             power = 0.9, type="two.sample", alternative="two.sided")
```

Two-sample t test power calculation

```
n = 110
delta = 0.18
sd = 0.41
sig.level = 0.05
power = 0.9
alternative = two.sided
```

NOTE: n is number in \*each\* group

In all, **220 people** would be required in the independent samples study (110 in each exposure group), as compared to only **57 people** (each measured twice) in the paired study.



## Chapter 28

# Comparing 3 or more Population Means: Analysis of Variance

Recall the National Youth Fitness Survey, which we explored a small piece of in some detail back in Section 7. We'll look at a different part of the same survey here - specifically the 280 children whose data are captured in the `nyfs2` file.

```
nyfs2
```

```
# A tibble: 280 x 21
  subject.id   sex age.exam      race.eth english
  <int> <fctr> <int>          <fctr>    <int>
1     73228 Male     4 5 Other or Multi-Race     1
2     72393 Male     4 2 Non-Hispanic Black     1
3     73303 Male     3 2 Non-Hispanic Black     1
4     72786 Male     5 1 Non-Hispanic White     1
5     73048 Male     3 2 Non-Hispanic Black     1
6     72556 Female   4 2 Non-Hispanic Black     1
7     72580 Female   5 2 Non-Hispanic Black     1
8     72532 Female   4        4 Other Hispanic   0
9     73012 Male     4 1 Non-Hispanic White     1
10    72099 Male     6 1 Non-Hispanic White     1
# ... with 270 more rows, and 16 more variables: income.cat3 <fctr>,
#   income.detail <fctr>, inc.to.pov <dbl>, weight.kg <dbl>,
#   height.cm <dbl>, bmi <dbl>, bmi.group <int>, bmi.cat <fctr>,
#   arm.length <dbl>, arm.circ <dbl>, waist.circ <dbl>, calf.circ <dbl>,
#   calf.skinfold <dbl>, triceps.skinfold <dbl>, subscap.skinfold <dbl>,
#   GMQ <int>
```

### 28.1 Comparing Gross Motor Quotient Scores by Income Level (3 Categories)

```
nyfs2a <- nyfs2 %>%
  select(subject.id, income.cat3, GMQ) %>%
  arrange(subject.id)
```

In this first analysis, we'll compare the population mean on the Gross Motor Quotient evaluation of

these kids across three groups defined by income level. Higher values of this GMQ measure indicate improved levels of gross motor development, both in terms of locomotor and object control. See [https://www.cdc.gov/Nchs/Nnyfs/Y\\_GMX.htm](https://www.cdc.gov/Nchs/Nnyfs/Y_GMX.htm) for more details.

```
nyfs2a %>%
  group_by(income.cat3) %>%
  summarise(n = n(), mean(GMQ), median(GMQ))
```

```
# A tibble: 3 x 4
  income.cat3     n `mean(GMQ)` `median(GMQ)`
  <fctr> <int>      <dbl>        <dbl>
1 High (65K or more)    92      95.7         97
2 Low (below 25K)      98      97.0         97
3 Middle (25 - 64K)    90      95.4         94
```

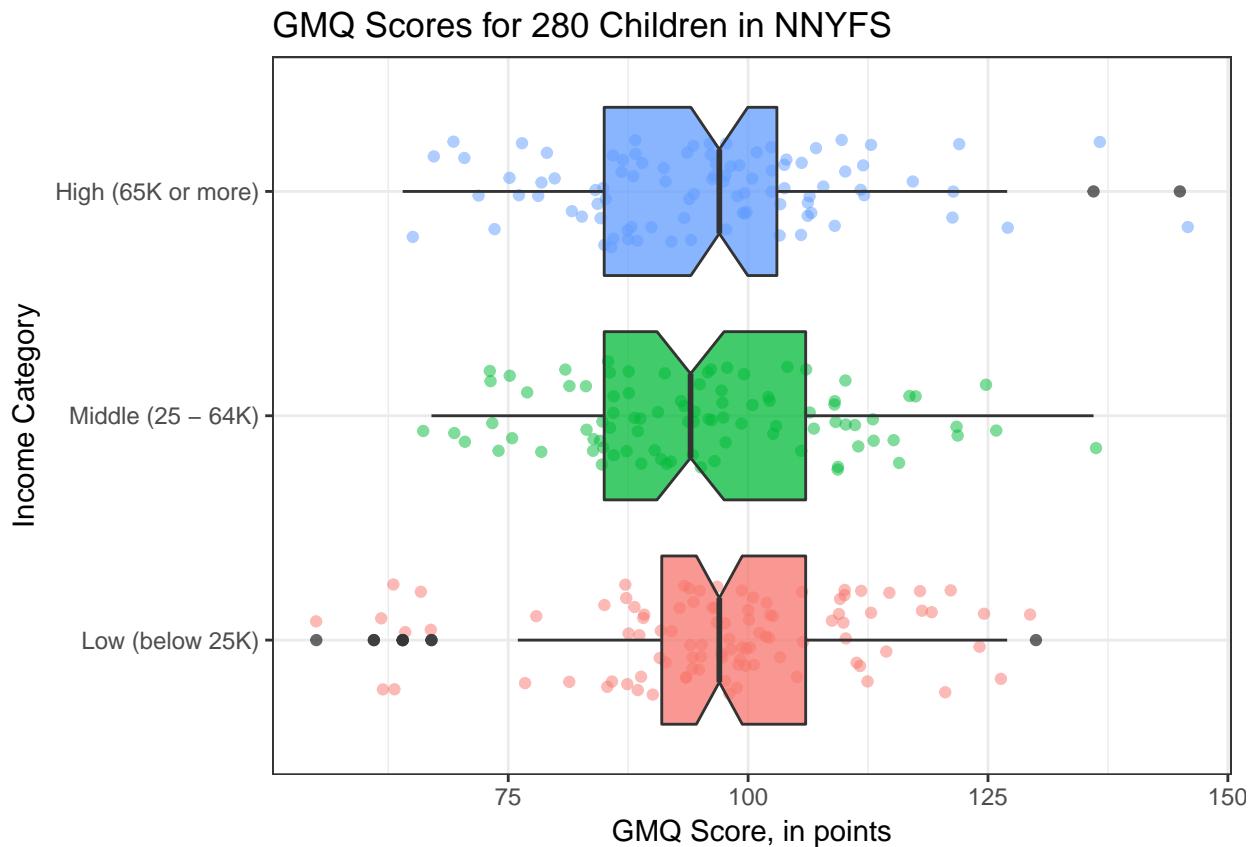
Uh, oh. We should rearrange those income categories to match a natural order from low to high.

```
nyfs2a$income.cat3 <-
  forcats::fct_relevel(nyfs2a$income.cat3,
                        "Low (below 25K)",
                        "Middle (25 - 64K)",
                        "High (65K or more)")
```

### 28.1.1 Graphical Summaries

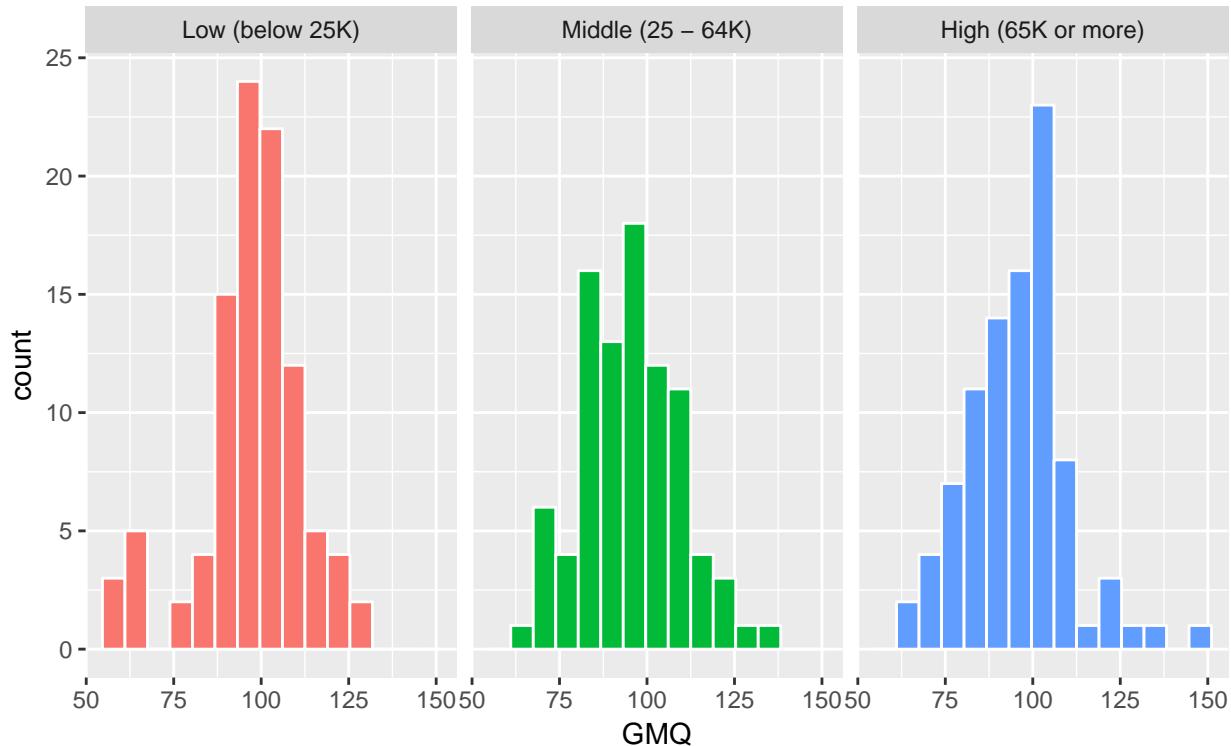
When working with three independent samples, I use graphs analogous to those we built for two independent samples.

```
ggplot(nyfs2a, aes(x = income.cat3, y = GMQ, fill = income.cat3)) +
  geom_jitter(aes(color = income.cat3), alpha = 0.5, width = 0.25) +
  geom_boxplot(notch = TRUE, alpha = 0.75) +
  theme_bw() +
  coord_flip() +
  guides(fill = FALSE, col = FALSE) +
  labs(title = "GMQ Scores for 280 Children in NNYFS",
       y = "GMQ Score, in points", x = "Income Category")
```



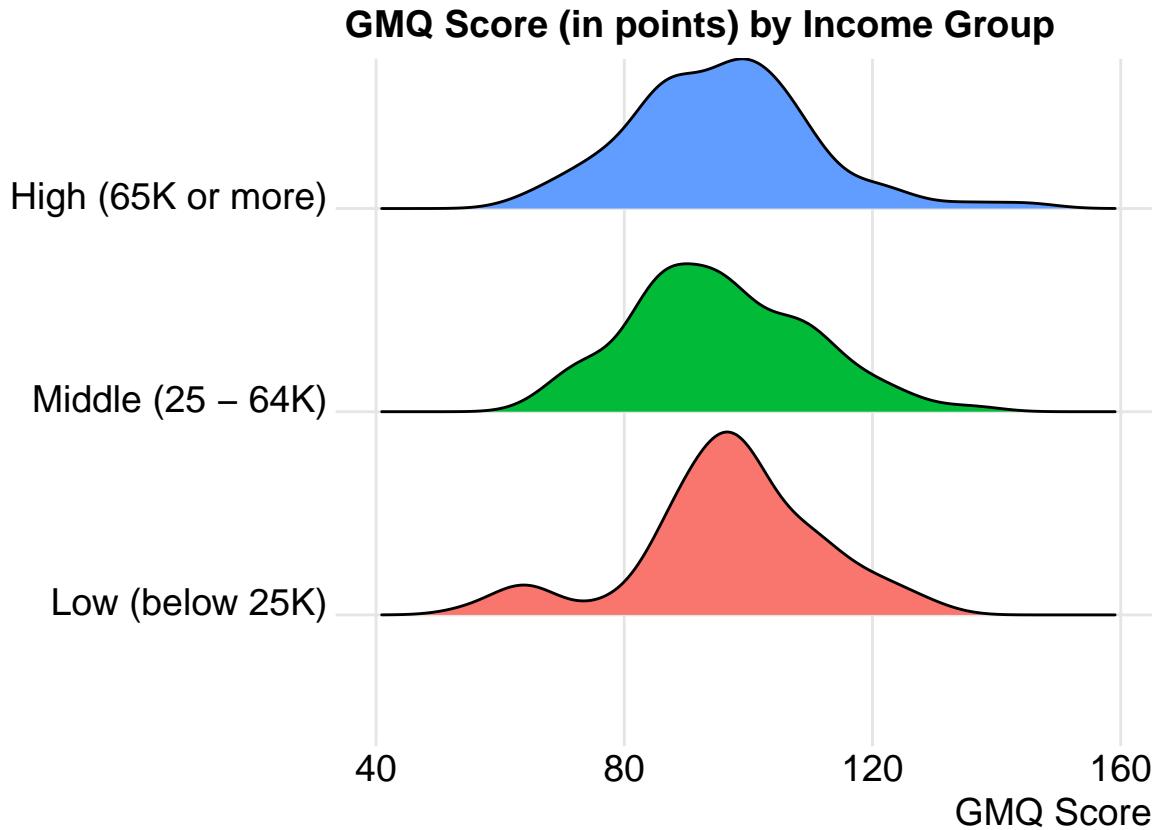
In addition to this comparison boxplot, we might consider faceted plots, like these histograms.

```
ggplot(nyfs2a, aes(x = GMQ, fill = income.cat3)) +
  geom_histogram(bins = 15, col = "white") +
  guides(fill = FALSE) +
  facet_wrap(~ income.cat3)
```



Or, if we want to ignore the (modest) sample size differences, we might consider density functions, perhaps through a ridgeline plot.

```
ggplot(nyfs2a, aes(x = GMQ, y = income.cat3, fill = income.cat3)) +
  ggridges::geom_density_ridges(scale = 0.9) +
  guides(fill = FALSE) +
  labs(title = "GMQ Score (in points) by Income Group",
       x = "GMQ Score", y = "") +
  ggridges::theme_ridges()
```



### 28.1.2 Numerical Summaries

```
by(nyfs2a$GMQ, nyfs2a$income.cat3, mosaic::favstats)
```

```
nyfs2a$income.cat3: Low (below 25K)
  min   Q1   median   Q3   max   mean   sd   n missing
    55    91      97   106   130    97  14.8  98      0
```

```
nyfs2a$income.cat3: Middle (25 – 64K)
  min   Q1   median   Q3   max   mean   sd   n missing
    67    85      94   106   136  95.4  14.2  90      0
```

```
nyfs2a$income.cat3: High (65K or more)
  min   Q1   median   Q3   max   mean   sd   n missing
    64    85      97   103   145  95.7  14.5  92      0
```

## 28.2 Alternative Procedures for Comparing More Than Two Means

Now, if we only had two independent samples, we'd be choosing between a pooled t test, a Welch t test, and a non-parametric procedure like the Wilcoxon-Mann-Whitney rank sum test, or even perhaps a bootstrap alternative.

In the case of more than two independent samples, we have methods analogous to the Welch test, and the rank sum test, and even the bootstrap, but we're going to be far more likely to select the **analysis of variance** (ANOVA) or an equivalent regression-based approach. These are the extensions of the pooled t test. Unless the sample outcome data are very clearly not Normally distributed, and no transformation is available which makes them appear approximately Normal in all of the groups we are comparing, we will stick with ANOVA.

### 28.2.1 Extending the Welch Test to $> 2$ Independent Samples

It is possible to extend the Welch two-sample t test (not assuming equal population variances) into an analogous one-factor analysis for comparing population means based on independent samples from more than two groups.

If we want to compare the population mean GMQ levels across those three income groups without assuming equal population variances, `oneway.test` is up to the task. The hypotheses being tested here are:

- $H_0: \mu_{Low} = \mu_{Middle} = \mu_{High}$  vs.
- $H_A:$  At least one of the population means is different than the others.

```
oneway.test(GMQ ~ income.cat3, data = nyfs2a)
```

```
One-way analysis of means (not assuming equal variances)
```

```
data: GMQ and income.cat3
F = 0.3, num df = 2, denom df = 200, p-value = 0.7
```

At our usual 5% significance level, since the  $p$  value for the one-way test is 0.71, we'd conclude that we cannot reject  $H_0$  and can only conclude that there is no significant difference between the true mean GMQ levels across the three income categories. You'll note that this isn't much help, though, because we don't have any measure of effect size, nor do we have any confidence intervals.

Like the analogous Welch t test, this approach allows us to forego the assumption of equal population variances in each of the three income groups, but it still requires us to assume that the populations are Normally distributed.

That said, most of the time when we have more than two levels of the factor of interest, we won't bother worrying about the equal population variance assumption, and will just use the one-factor ANOVA approach (with pooled variances) described below, to make the comparisons of interest.

### 28.2.2 Extending the Rank Sum Test to $> 2$ Independent Samples

It is also possible to extend the Wilcoxon-Mann-Whitney two-sample test into an analogous one-factor analysis called the **Kruskal-Wallis test** for comparing population measures of location based on independent samples from more than two groups.

If we want to compare the centers of the distributions of population GMQ score across our three income groups without assuming Normality, we can use `kruskal.test`.

The hypotheses being tested here are still as before, but the  $\mu$  referred to here is a measure of location other than the population mean:

- $H_0: \mu_{Low} = \mu_{Middle} = \mu_{High}$  vs.
- $H_A:$  At least one of the population means is different than the others.

```
kruskal.test(GMQ ~ income.cat3, data = nyfs2a)
```

```
Kruskal-Wallis rank sum test
```

```
data: GMQ by income.cat3
Kruskal-Wallis chi-squared = 2, df = 2, p-value = 0.3
```

So, in this case, at our usual 5% significance level, since the  $p$  value for the Kruskal-Wallis test is 0.31, we'd conclude that we cannot reject  $H_0$  and can only conclude that there is no significant difference between the true mean GMQ scores across the three income categories. Again, note that this isn't much help, though, because we don't have any measure of effect size, nor do we have any confidence intervals.

That said, most of the time when we have more than two levels of the factor of interest, we won't bother worrying about potential violations of the Normality assumption unless they are glaring, and will just use the usual one-factor ANOVA approach (with pooled variances) described below, to make the comparisons of interest.

### 28.2.3 Can we use the bootstrap to compare more than two means?

Sure. There are both ANOVA and ANCOVA analogues using the bootstrap, and in fact, there are power calculations based on the bootstrap, too.

We'll discuss all of these methods in 432, but if you want to see some example code, look at <https://sammancuso.com/2015/05/28/bootstrap-anova-with-pairwise-post-hoc-contrasts/>

## 28.3 The Analysis of Variance

Extending the two-sample t test (assuming equal population variances) into a comparison of more than two samples uses the **analysis of variance** or ANOVA.

This is analysis of a continuous outcome variable on the basis of a single categorical factor, in fact, it's often called one-factor ANOVA or one-way ANOVA to indicate that the outcome is being split up into the groups defined by a single factor.

The null hypothesis is that the population means are all the same, and the alternative is that this is not the case. When there are just two groups, then this boils down to an F test that is equivalent to the Pooled t test.

### 28.3.1 The `oneway.test` approach

R will produce some elements of a one-factor ANOVA using the `oneway.test` command:

```
oneway.test(GMQ ~ income.cat3, data = nyfs2a, var.equal=TRUE)
```

```
One-way analysis of means
```

```
data: GMQ and income.cat3
F = 0.3, num df = 2, denom df = 300, p-value = 0.7
```

This isn't the full analysis, though, which would require a more complete ANOVA table. There are two equivalent approaches to obtaining the full ANOVA table when comparing a series of 2 or more population means based on independent samples.

### 28.3.2 Using the `aov` approach and the `summary` function

Here's one possible ANOVA table, which doesn't require directly fitting a linear model.

```
summary(aov(GMQ ~ income.cat3, data = nyfs2a))
```

	Df	Sum Sq	Mean Sq	F value	Pr(>F)
income.cat3	2	146	72.8	0.35	0.71
Residuals	277	58174	210.0		

### 28.3.3 Using the anova function after fitting a linear model

An equivalent way to get identical results in a slightly different format runs the linear model behind the ANOVA approach directly.

```
anova(lm(GMQ ~ income.cat3, data = nyfs2a))
```

Analysis of Variance Table

Response: GMQ

	Df	Sum Sq	Mean Sq	F value	Pr(>F)
income.cat3	2	146	72.8	0.35	0.71
Residuals	277	58174	210.0		

## 28.4 Interpreting the ANOVA Table

### 28.4.1 What are we Testing?

The null hypothesis for the ANOVA table is that the population means of the outcome across the various levels of the factor of interest are all the same, against a two-sided alternative hypothesis that the level-specific population means are not all the same.

Specifically, if we have a grouping factor with  $k$  levels, then we are testing:

- $H_0: \mu_1 = \mu_2 = \dots = \mu_k$  vs.
- $H_A: \text{At least one of the population means } \mu_1, \mu_2, \dots, \mu_k \text{ is different from the others.}$

### 28.4.2 Elements of the ANOVA Table

The ANOVA table breaks down the variation in the outcome explained by the  $k$  levels of the factor of interest, and the variation in the outcome which remains (the Residual, or Error).

Specifically, the elements of the ANOVA table are:

1. the degrees of freedom (labeled Df) for the factor of interest and for the Residuals
2. the sums of squares (labeled Sum Sq) for the factor of interest and for the Residuals
3. the mean square (labeled Mean Sq) for the factor of interest and for the Residuals
4. the ANOVA F test statistic (labeled F value), which is used to generate
5. the  $p$  value for the comparison assessed by the ANOVA model, labeled Pr(>F)

### 28.4.3 The Degrees of Freedom

```
anova(lm(GMQ ~ income.cat3, data = nyfs2a))
```

## Analysis of Variance Table

```
Response: GMQ
          Df Sum Sq Mean Sq F value Pr(>F)
income.cat3    2     146    72.8    0.35   0.71
Residuals    277  58174   210.0
```

- The **degrees of freedom** attributable to the factor of interest (here, Income category) is the number of levels of the factor minus 1. Here, we have three Income categories (levels), so  $df(income.cat3) = 2$ .
- The total degrees of freedom are the number of observations (across all levels of the factor) minus 1. We have 280 GMQ scores in the `nyfs2a` data, so the  $df(\text{Total})$  must be 279, although the Total row isn't shown by R in its output.
- The Residual degrees of freedom are the Total df - Factor df. So, here, that's  $279 - 2 = 277$ .

## 28.4.4 The Sums of Squares

```
anova(lm(GMQ ~ income.cat3, data = nyfs2a))
```

## Analysis of Variance Table

```
Response: GMQ
          Df Sum Sq Mean Sq F value Pr(>F)
income.cat3    2     146    72.8    0.35   0.71
Residuals    277  58174   210.0
```

- The sum of squares (often abbreviated SS or Sum Sq) represents variation explained.
- The factor SS is the sum across all levels of the factor of the sample size for the level multiplied by the squared difference between the level mean and the overall mean across all levels. Here,  $SS(income.cat3) = 146$
- The total SS is the sum across all observations of the square of the difference between the individual values and the overall mean. Here, that is  $146 + 58174 = 58320$
- Residual SS = Total SS - Factor SS.
- Also of interest is a calculation called  $\eta^2$ , (“eta-squared”), which is equivalent to  $R^2$  in a linear model.
  - $\eta^2 = SS(\text{Factor})/SS(\text{Total})$  = the proportion of variation in our outcome (here, GMQ) explained by the variation between groups (here, income groups)
  - In our case,  $\eta^2 = 146 / (146 + 58174) = 146 / 58320 = 0.0025$
  - So, Income Category alone accounts for about 0.25% of the variation in GMQ levels observed in these data.

## 28.4.5 The Mean Square

```
anova(lm(GMQ ~ income.cat3, data = nyfs2a))
```

## Analysis of Variance Table

```
Response: GMQ
          Df Sum Sq Mean Sq F value Pr(>F)
income.cat3    2     146    72.8    0.35   0.71
Residuals    277  58174   210.0
```

- The Mean Square is the Sum of Squares divided by the degrees of freedom, so  $MS(\text{Factor}) = SS(\text{Factor})/df(\text{Factor})$ .

- In our case,  $MS(income.cat3) = SS(income.cat3)/df(income.cat3) = 146 / 2 = 72.848$  (notice that R maintains more decimal places than it shows for these calculations) and
- $MS(\text{Residuals}) = SS(\text{Residuals}) / df(\text{Residuals}) = 58174 / 277 = 210.014$ .
  - $MS(\text{Residuals})$  or  $MS(\text{Error})$  is an estimate of the residual variance which corresponds to  $\sigma^2$  in the underlying linear model for the outcome of interest, here GMQ.

### 28.4.6 The F Test Statistic and $p$ Value

```
anova(lm(GMQ ~ income.cat3, data = nyfs2a))
```

Analysis of Variance Table

Response: GMQ

	Df	Sum Sq	Mean Sq	F value	Pr(>F)
income.cat3	2	146	72.8	0.35	0.71
Residuals	277	58174	210.0		

- The ANOVA F test is obtained by calculating  $MS(\text{Factor}) / MS(\text{Residuals})$ . So in our case,  $F = 72.848 / 210.014 = 0.3469$
- The F test statistic is then compared to a specific F distribution to obtain a  $p$  value, which is shown here to be 0.7072
- Specifically, the observed F test statistic is compared to an F distribution with numerator df = Factor df, and denominator df = Residual df to obtain the  $p$  value.
  - Here, we have  $SS(\text{Factor}) = 146$  (approximately), and  $df(\text{Factor}) = 2$ , leaving  $MS(\text{Factor}) = 72.848$
  - We have  $SS(\text{Residual}) = 58174$ , and  $df(\text{Residual}) = 277$ , leaving  $MS(\text{Residual}) = 210.014$
  - $MS(\text{Factor}) / MS(\text{Residual}) = F \text{ value} = 0.3469$ , which, when compared to an F distribution with 2 and 277 degrees of freedom, yields a  $p$  value of 0.7072

## 28.5 The Residual Standard Error

The residual standard error is simply the square root of the variance estimate  $MS(\text{Residual})$ . Here,  $MS(\text{Residual}) = 210.014$ , so the Residual standard error = 14.49 points.

## 28.6 The Proportion of Variance Explained by the Factor, or $\eta^2$

We will often summarize the proportion of the variation explained by the factor. The summary statistic is called eta-squared ( $\eta^2$ ), and is equivalent to the  $R^2$  value we have seen previously in linear regression models.

Again,  $\eta^2 = SS(\text{Factor}) / SS(\text{Total})$

Here, we have -  $SS(income.cat3) = 146$  and  $SS(\text{Residuals}) = 58174$ , so  $SS(\text{Total}) = 58320$  - Thus,  $\eta^2 = SS(\text{Factor})/SS(\text{Total}) = 146/58320 = 0.0025$

The income category accounts for 0.25% of the variation in GMQ levels: only a tiny fraction.

## 28.7 The Regression Approach to Compare Population Means based on Independent Samples

This approach is equivalent to the ANOVA approach, and thus also (when there are just two samples to compare) to the pooled-variance t test. We run a linear regression model to predict the outcome (here, GMQ)

on the basis of the categorical factor with three levels (here, `income.cat3`)

```
summary(lm(GMQ ~ income.cat3, data=nyfs2a))
```

Call:

```
lm(formula = GMQ ~ income.cat3, data = nyfs2a)
```

Residuals:

Min	1Q	Median	3Q	Max
-42.03	-9.03	-0.03	8.97	49.27

Coefficients:

	Estimate	Std. Error	t value	Pr(> t )
(Intercept)	97.03	1.46	66.28	<2e-16 ***
income.cat3Middle (25 - 64K)	-1.66	2.12	-0.79	0.43
income.cat3High (65K or more)	-1.30	2.10	-0.62	0.54
---				
Signif. codes:	0 '***'	0.001 '**'	0.01 '*'	0.05 '.'
	0.1	' '	1	

Residual standard error: 14.5 on 277 degrees of freedom

Multiple R-squared: 0.0025, Adjusted R-squared: -0.0047

F-statistic: 0.347 on 2 and 277 DF, p-value: 0.707

## 28.7.1 Interpreting the Regression Output

This output tells us many things, but for now, we'll focus just on the coefficients output, which tells us that:

- the point estimate for the population mean GMQ score across “Low” income subjects is 97.03
- the point estimate (sample mean difference) for the difference in population mean GMQ level between the “Middle” and “Low” income subjects is -1.66 (in words, the Middle income kids have lower GMQ scores than the Low income kids by 1.66 points on average.)
- the point estimate (sample mean difference) for the difference in population mean GMQ level between the “High” and “Low” income subjects is -1.30 (in words, the High income kids have lower GMQ scores than the Low income kids by 1.30 points on average.)

Of course, we knew all of this already from a summary of the sample means.

```
nyfs2a %>%
  group_by(income.cat3) %>%
  summarise(n = n(), mean(GMQ))
```

```
# A tibble: 3 x 3
  income.cat3     n `mean(GMQ)`
  <fctr> <int>     <dbl>
1 Low (below 25K)    98      97.0
2 Middle (25 - 64K)   90      95.4
3 High (65K or more)  92      95.7
```

The model for predicting GMQ is based on two binary (1/0) indicator variables, specifically, we have:

- Estimated GMQ = 97.03 - 1.66 x [1 if Middle income or 0 if not] - 1.30 x [1 if High income or 0 if not]

The coefficients section also provides a standard error and t statistic and two-sided *p* value for each coefficient.

### 28.7.2 The Full ANOVA Table

To see the full ANOVA table corresponding to any linear regression model, we run...

```
anova(lm(GMQ ~ income.cat3, data=nyfs2a))
```

Analysis of Variance Table

Response: GMQ

	Df	Sum Sq	Mean Sq	F value	Pr(>F)
income.cat3	2	146	72.8	0.35	0.71
Residuals	277	58174	210.0		

### 28.7.3 ANOVA Assumptions

The assumptions behind analysis of variance are the same as those behind a linear model. Of specific interest are:

- The samples obtained from each group are independent.
- Ideally, the samples from each group are a random sample from the population described by that group.
- In the population, the variance of the outcome in each group is equal. (This is less of an issue if our study involves a balanced design.)
- In the population, we have Normal distributions of the outcome in each group.

Happily, the F test is fairly robust to violations of the Normality assumption.

## 28.8 Equivalent approach to get ANOVA Results

$H_0: \mu_{Low} = \mu_{Middle} = \mu_{High}$  vs.  $H_A: H_0$  not true.

```
summary(aov(GMQ ~ income.cat3, data = nyfs2a))
```

	Df	Sum Sq	Mean Sq	F value	Pr(>F)
income.cat3	2	146	72.8	0.35	0.71
Residuals	277	58174	210.0		

So which of the pairs of means are significantly different?

## 28.9 The Problem of Multiple Comparisons

1. Suppose we compare High to Low, using a test with  $\alpha = 0.05$
2. Then we compare Middle to Low on the same outcome, also using  $\alpha = 0.05$
3. Then we compare High to Middle, also with  $\alpha = 0.05$

What is our overall  $\alpha$  level across these three comparisons?

- It could be as bad as  $0.05 + 0.05 + 0.05$ , or 0.15.
- Rather than our nominal 95% confidence, we have something as low as 85% confidence across this set of simultaneous comparisons.

### 28.9.1 The Bonferroni solution

1. Suppose we compare High to Low, using a test with  $\alpha = 0.05/3$

2. Then we compare Middle to Low on the same outcome, also using  $\alpha = 0.05/3$
3. Then we compare High to Middle, also with  $\alpha = 0.05/3$

Then across these three comparisons, our overall  $\alpha$  can be (at worst)

- $0.05/3 + 0.05/3 + 0.05/3 = 0.05$
- So by changing our nominal confidence level from 95% to 98.333% in each comparison, we wind up with at least 95% confidence across this set of simultaneous comparisons.
- This is a conservative (worst case) approach.

Goal: Simultaneous  $p$  values comparing White vs AA, AA vs Other and White vs Other

```
pairwise.t.test(nyfs2a$GMQ, nyfs2a$income.cat3, p.adjust="bonferroni")
```

Pairwise comparisons using t tests with pooled SD

```
data: nyfs2a$GMQ and nyfs2a$income.cat3

Low (below 25K) Middle (25 - 64K)
Middle (25 - 64K) 1 -
High (65K or more) 1 1
```

P value adjustment method: bonferroni

In this case, none of these  $p$  values are even remotely close to being small enough to indicate statistical significance. They're all 1, in effect.

## 28.9.2 Pairwise Comparisons of Group Means using Tukey's Honestly Significant Differences

Goal: Simultaneous (less conservative) confidence intervals and  $p$  values for our three pairwise comparisons (High vs. Low, High vs. Middle, Middle vs. Low)

```
TukeyHSD(aov(GMQ ~ income.cat3, data = nyfs2a))
```

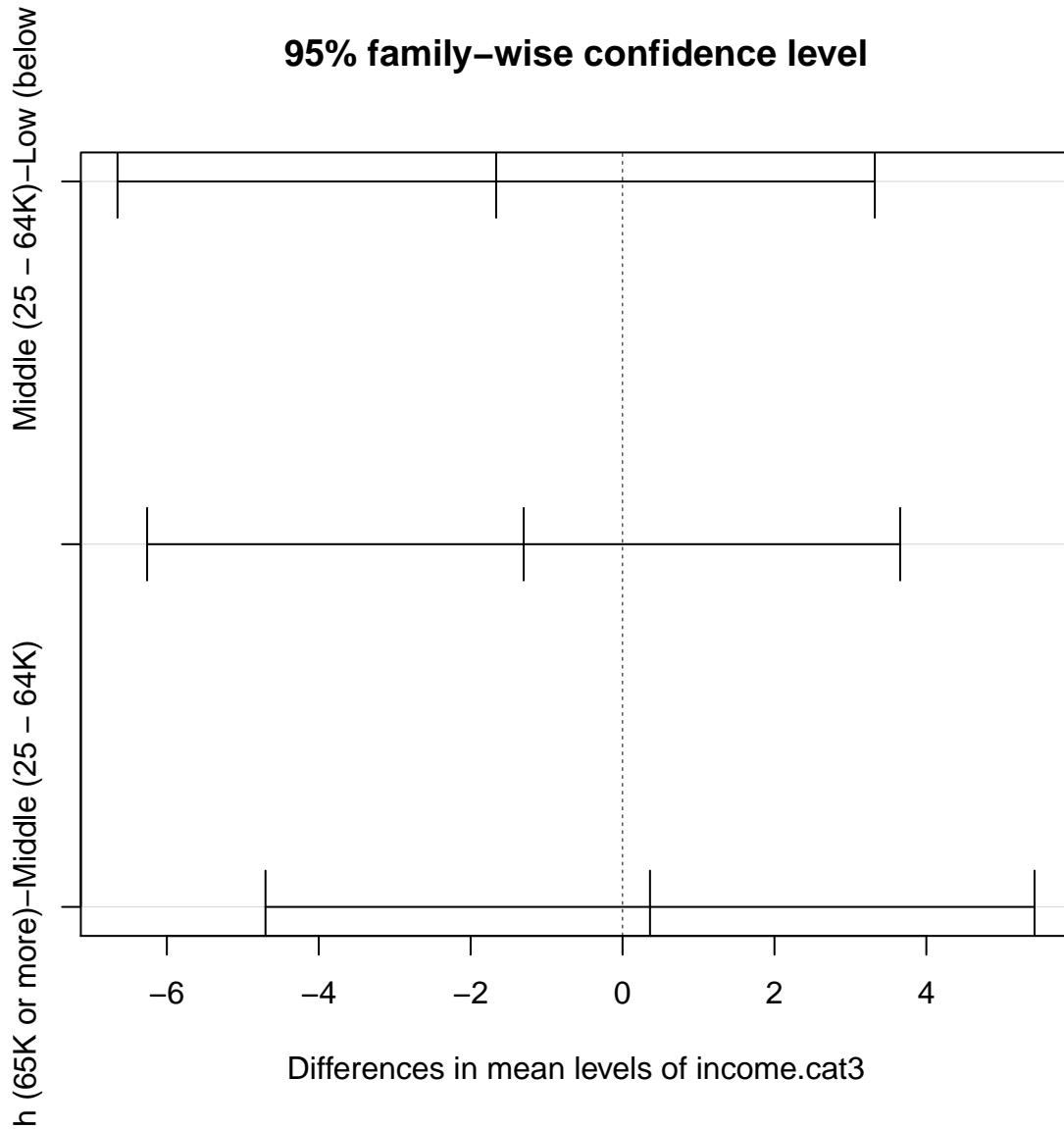
```
Tukey multiple comparisons of means
95% family-wise confidence level

Fit: aov(formula = GMQ ~ income.cat3, data = nyfs2a)

$income.cat3
            diff   lwr   upr p adj
Middle (25 - 64K)-Low (below 25K) -1.664 -6.65 3.32 0.712
High (65K or more)-Low (below 25K) -1.302 -6.26 3.65 0.810
High (65K or more)-Middle (25 - 64K)  0.362 -4.70 5.42 0.985
```

## 28.9.3 Plotting the Tukey HSD results

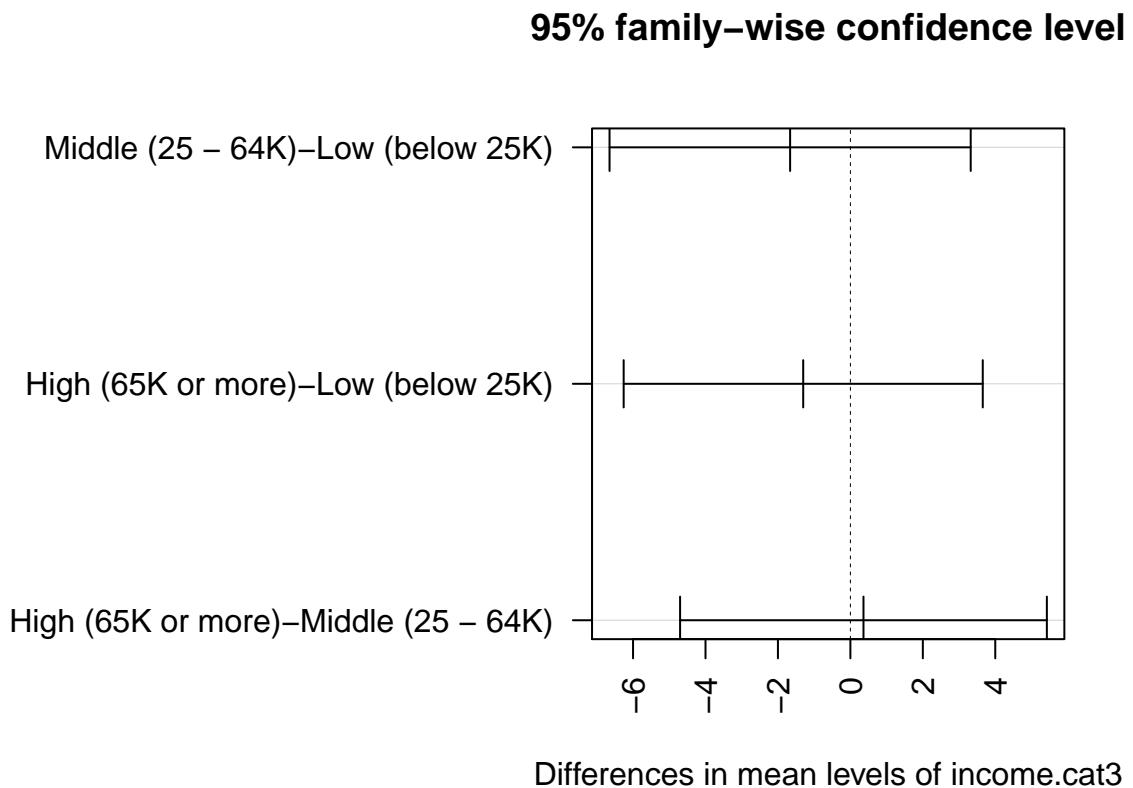
```
plot(TukeyHSD(aov(GMQ ~ income.cat3, data = nyfs2a)))
```



Note that the default positioning of the y axis in the plot of Tukey HSD results can be problematic. If we have longer names, in particular, for the levels of our factor, R will leave out some of the labels. We can alleviate that problem either by using the `fct_recode` function in the `forcats` package to rename the factor levels, or we can use the following code to reconfigure the margins of the plot.

```
mar.default <- c(5, 6, 4, 2) + 0.1 # save default plotting margins

par(mar = mar.default + c(0, 12, 0, 0))
plot(TukeyHSD(aov(GMQ ~ income.cat3, data = nyfs2a)), las = 2)
```



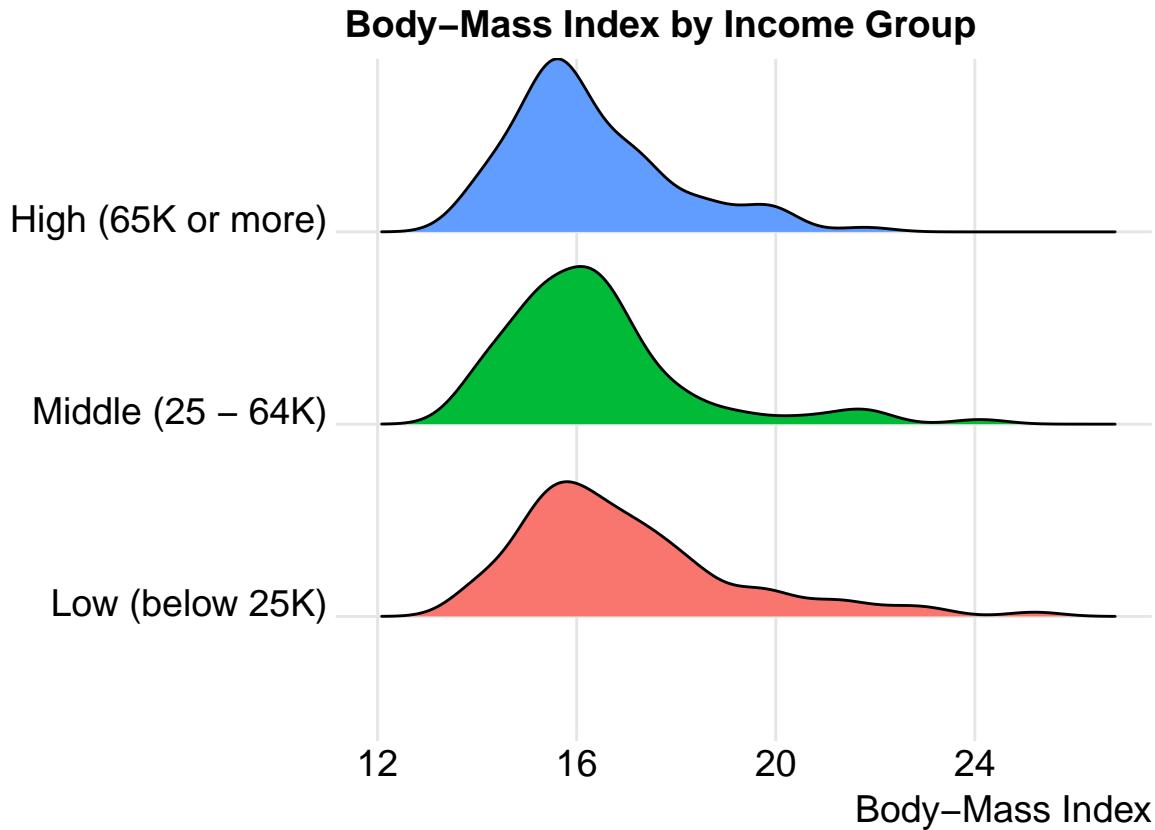
```
par(mar = mar.default) # return to normal plotting margins
```

## 28.10 What if we consider another outcome, BMI?

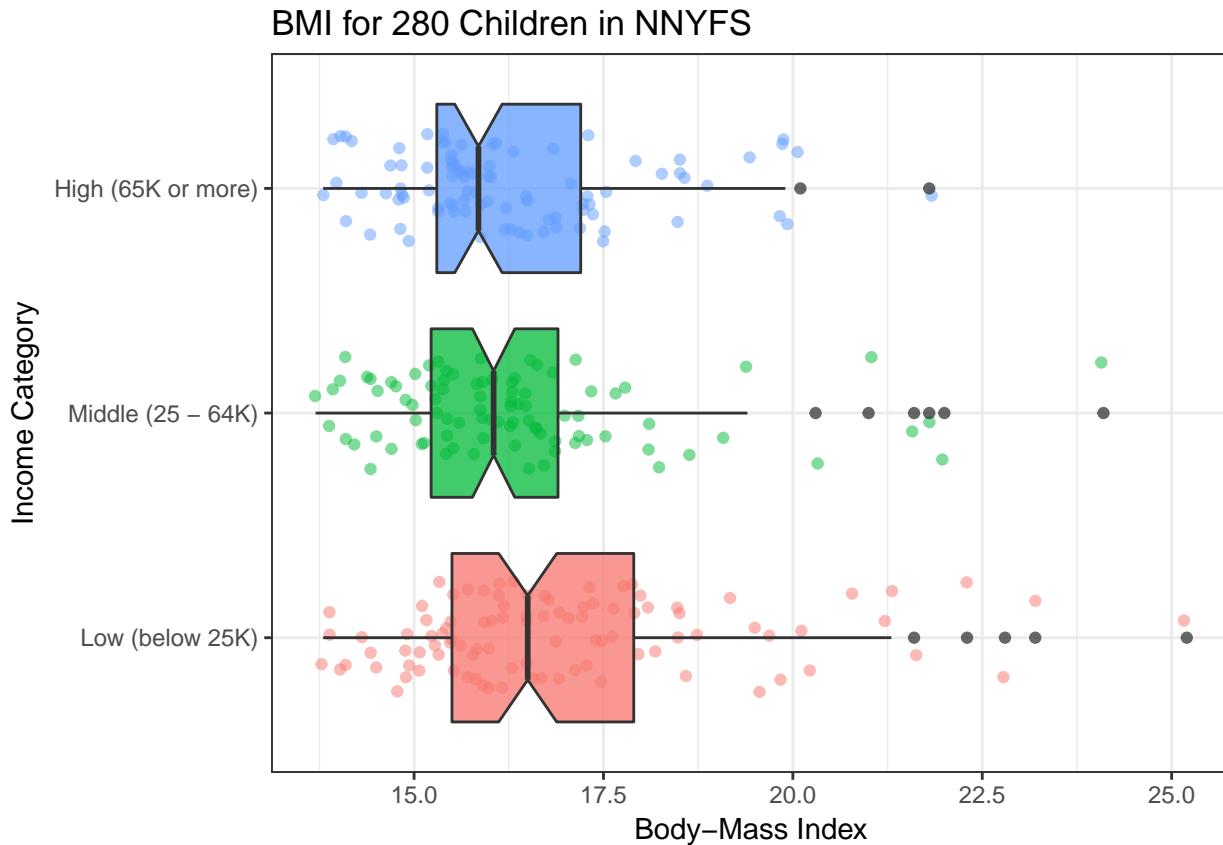
We'll look at the full data set in `nyfs2` now, so we can look at BMI as a function of income.

```
nyfs2$income.cat3 <-
  forcats::fct_relevel(nyfs2$income.cat3,
    "Low (below 25K)",
    "Middle (25 – 64K)",
    "High (65K or more)")

ggplot(nyfs2, aes(x = bmi, y = income.cat3, fill = income.cat3)) +
  ggridges::geom_density_ridges(scale = 0.9) +
  guides(fill = FALSE) +
  labs(title = "Body-Mass Index by Income Group",
    x = "Body-Mass Index", y = "") +
  ggridges::theme_ridges()
```



```
ggplot(nyfs2, aes(x = income.cat3, y = bmi, fill = income.cat3)) +
  geom_jitter(aes(color = income.cat3), alpha = 0.5, width = 0.25) +
  geom_boxplot(notch = TRUE, alpha = 0.75) +
  theme_bw() +
  coord_flip() +
  guides(fill = FALSE, col = FALSE) +
  labs(title = "BMI for 280 Children in NNYFS",
       y = "Body-Mass Index", x = "Income Category")
```



Here are the descriptive numerical summaries:

```
by(nyfs2$bmi, nyfs2$income.cat3, mosaic::favstats)
```

```
nyfs2$income.cat3: Low (below 25K)
  min   Q1 median   Q3 max mean   sd n missing
 13.8 15.5 16.5 17.9 25.2 17 2.19 98      0
```

```
nyfs2$income.cat3: Middle (25 - 64K)
  min   Q1 median   Q3 max mean   sd n missing
 13.7 15.2 16.1 16.9 24.1 16.4 1.9 90      0
```

```
nyfs2$income.cat3: High (65K or more)
  min   Q1 median   Q3 max mean   sd n missing
 13.8 15.3 15.9 17.2 21.8 16.3 1.61 92      0
```

Here is the ANOVA table.

```
anova(lm(bmi ~ income.cat3, data = nyfs2))
```

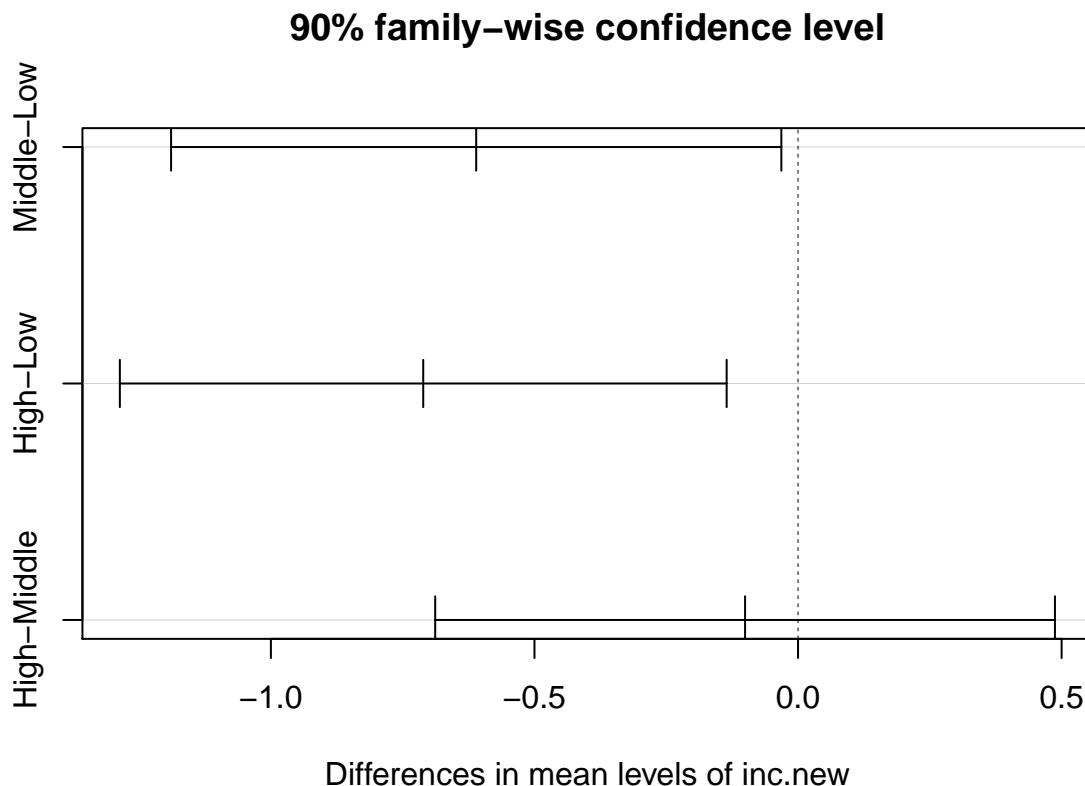
Analysis of Variance Table

```
Response: bmi
          Df Sum Sq Mean Sq F value Pr(>F)
income.cat3  2     28    14.2    3.83  0.023 *
Residuals 277 1025     3.7
---
Signif. codes:  0 '***' 0.001 '**' 0.01 '*' 0.05 '.' 0.1 ' ' 1
```

And we see a statistically significant result at usual  $\alpha$  levels. Let's consider the Tukey HSD results. First, we'll create a factor with shorter labels.

```
nyfs2$inc.new <-
  forcats::fct_recode(nyfs2$income.cat3,
    "Low" = "Low (below 25K)",
    "Middle" = "Middle (25 - 64K)",
    "High" = "High (65K or more)")

plot(TukeyHSD(aov(bmi ~ inc.new, data = nyfs2),
  conf.level = 0.90))
```



It appears that there is a statistically significant difference between the `bmi` means of the “Low” group and both the “High” and “Middle” group at the 10% significance level, but no significant difference between “Middle” and “High.” Details of those  $p$  values and confidence intervals for those pairwise comparisons follow.

```
TukeyHSD(aov(bmi ~ inc.new, data = nyfs2),
  conf.level = 0.90)
```

```
Tukey multiple comparisons of means
90% family-wise confidence level

Fit: aov(formula = bmi ~ inc.new, data = nyfs2)

$inc.new
      diff     lwr      upr p adj
Middle-Low -0.611 -1.189 -0.0317 0.078
High-Low    -0.711 -1.287 -0.1354 0.031
```

High-Middle -0.100 -0.688 0.4874 0.934



# Chapter 29

## Estimating a Population Rate or Proportion

We've focused on creating statistical inferences about a population mean, or difference between means, where we care about a quantitative outcome. Now, we'll tackle **categorical** outcomes. We'll start by estimating a confidence interval around a population proportion.

### 29.1 Ebola Mortality Rates through 9 Months of the Epidemic

The World Health Organization's Ebola Response Team published an article<sup>1</sup> in the October 16, 2014 issue of the New England Journal of Medicine, which contained some data I will use in this example, focusing on materials from their Table 2.

As of September 14, 2014, a total of 4,507 confirmed and probable cases of Ebola virus disease (EVD) had been reported from West Africa. In our example, we will look at a set of 1,737 cases, with definitive outcomes, reported in Guinea, Liberia, Nigeria and Sierra Leone.

Across these 1,737 cases, a total of 1,229 cases led to death. Based on these sample data, what can be said about the case fatality rate in the population of EVD cases with definitive outcomes for this epidemic?

### 29.2 A $100(1-\alpha)\%$ Confidence Interval for a Population Proportion

Suppose we want to estimate a confidence interval for an unknown population proportion,  $\pi$ , on the basis of a random sample of  $n$  observations from that population which yields a sample proportion of  $p$ . Note that this  $p$  is the sample proportion – it's not a  $p$  value.

A  $100(1-\alpha)\%$  confidence interval for the population proportion  $\pi$  can be created by using the standard normal distribution, the sample proportion,  $p$ , and the standard error of a sample proportion, which is defined as the square root of  $p$  multiplied by  $(1-p)$  divided by the sample size,  $n$ .

Specifically, our confidence interval is  $p \pm Z_{\alpha/2} \sqrt{\frac{p(1-p)}{n}}$

---

<sup>1</sup>WHO Ebola Response Team (2014) Ebola virus disease in West Africa: The first 9 months of the epidemic and forward projections. *New Engl J Med* 371: 1481-1495 doi: 10.1056/NEJMoa1411100

where  $Z_{\alpha/2}$  = the value from a standard Normal distribution cutting off the top  $\alpha/2$  of the distribution, obtained in R by substituting the desired  $\alpha/2$  value into the following command: `qnorm(alpha/2, lower.tail=FALSE)`.

- Note: This interval is reasonably accurate so long as  $np$  and  $n(1-p)$  are each at least 5.

## 29.3 Working through the Ebola Virus Disease Example

In our case, we have  $n = 1,737$  subjects, of whom we observed death in 1,229, for a sample proportion of  $p = \frac{1229}{1737} = 0.708$ . The standard error of that sample proportion will be

$$SE(p) = \sqrt{\frac{p(1-p)}{n}} = \sqrt{\frac{0.708(1-0.708)}{1737}} = 0.011$$

And our 95% confidence interval (so that we'll use  $\alpha = 0.05$ ) for the true population proportion,  $\pi$ , of EVD cases with definitive outcomes, who will die is  $p \pm Z_{0.025} \sqrt{\frac{p(1-p)}{n}}$ , or  $0.708 \pm 1.96(0.011) = 0.708 \pm 0.022$ , or (0.686, 0.730)

Note that I simply recalled from our prior work that  $Z_{0.025} = 1.96$ , but we can verify this:

```
qnorm(0.025, lower.tail=FALSE)
```

```
[1] 1.96
```

Since both  $np=(1737)(0.708)=1230$  and  $n(1-p)=(1737)(1-0.708)=507$  are substantially greater than 5, this should be a reasonably accurate confidence interval.

We are 95% confident that the true population proportion is between 0.686 and 0.730. Equivalently, we could say that we're 95% confident that the true case fatality rate expressed as a percentage rather than a proportion, is between 68.6% and 73.0%.

I am aware of at least three different procedures for estimating a confidence interval for a population proportion using R. All have minor weaknesses: none is importantly different from the others in many practical situations.

## 29.4 The `prop.test` approach (Wald test)

The `prop.test` function can be used to establish a very similar confidence interval to the one we calculated above, based on something called the Wald test.

```
prop.test(x = 1229, n = 1737)
```

```
1-sample proportions test with continuity correction

data: 1229 out of 1737, null probability 0.5
X-squared = 300, df = 1, p-value <2e-16
alternative hypothesis: true p is not equal to 0.5
95 percent confidence interval:
 0.685 0.729
sample estimates:
      p 
0.708
```

The 95% confidence interval by this method is (0.685, 0.729), which is close, but not quite the same, to our original estimate of (0.686, 0.730.)

The difference from our calculated interval is attributable to differences in rounding, plus the addition of a continuity correction, since we are using a Normal approximation to the exact binomial distribution to establish our margin for error. R, by default, includes this continuity correction for this test.

## 29.5 The `binom.test` approach (Clopper and Pearson “exact” test)

The `binom.test` command can be used to establish an “exact” confidence interval. This uses the method of Clopper and Pearson from 1934, and is exact in the sense that it guarantees, for instance, that the confidence interval is at least 95%, but not that the interval isn’t wider than perhaps it needs to be.

```
binom.test(x = 1229, n = 1737)
```

Exact binomial test

```
data: 1229 and 1737
number of successes = 1000, number of trials = 2000, p-value
<2e-16
alternative hypothesis: true probability of success is not equal to 0.5
95 percent confidence interval:
0.686 0.729
sample estimates:
probability of success
0.708
```

The 95% confidence interval by this method is (0.686, 0.729), which is a little closer, but still not quite the same, as our original estimate of (0.686, 0.730.)

## 29.6 SAIFS: single augmentation with an imaginary failure or success

SAIFS stands for “single augmentation with an imaginary failure or success” and the method I’ll describe is one of several similar approaches. The next subsection describes the R code for calculating the relevant confidence interval.

An approach I like for the estimation of a confidence interval for a single population proportion/rate<sup>2</sup> is to estimate the lower bound of a confidence interval with an imaginary failure added to the observed data, and estimate the upper bound of a confidence interval with an imaginary success added to the data.

Suppose we have X successes in n trials, and we want to establish a confidence interval for the population proportion of successes.

Let  $p_1 = (X + 0)/(n + 1)$ ,  $p_2 = (X + 1)/(n + 1)$ ,  $q_1 = 1 - p_1$ ,  $q_2 = 1 - p_2$

- The lower bound of a  $100(1-\alpha)\%$  confidence interval for the population proportion of successes using the SAIFS procedure is then  $LB_{SAIFS}(x, n, \alpha) = p_1 - t_{\alpha/2, n-1} \sqrt{\frac{p_1 q_1}{n}}$
- The upper bound of that same  $100(1-\alpha)\%$  confidence interval for the population proportion of successes using the SAIFS procedure is  $UB_{SAIFS}(x, n, \alpha) = p_2 + t_{\alpha/2, n-1} \sqrt{\frac{p_2 q_2}{n}}$

---

<sup>2</sup>See Borkowf CB (2006) Constructing binomial confidence intervals with near nominal coverage by adding a single imaginary failure or success. Statistics in Medicine. 25(21): 3679-3695. doi: 10.1002/sim.2469, or get the whole PDF of the paper at <http://onlinelibrary.wiley.com/doi/10.1002/sim.2469/pdf>

## 29.7 Using the SAIFS Approach in the Ebola Example

Returning to the Ebola Virus Disease example, we've got 1229 "successes" (it's hard to think of death as a success, but from a calculation standpoint, that's what we're doing) out of 1737 "trials", so that  $X = 1229$  and  $n = 1737$ .

So we have  $p_1 = \frac{X+0}{n+1} = \frac{1229}{1738} = 0.7071$ ,  $p_2 = \frac{X+1}{n+1} = \frac{1230}{1738} = 0.7077$ , and  $q_1 = 1 - p_1 = 0.2929$  and  $q_2 = 1 - p_2 = 0.2923$

We have  $n = 1737$  so if we want a 95% confidence interval ( $\alpha = 0.05$ ), then we have  $t_{\alpha/2,n-1} = t_{0.025,1736} = 1.9613$ , which I determined using R's `qt` function:

```
qt(0.025, df = 1736, lower.tail=FALSE)
```

[1] 1.96

- Thus, our lower bound for a 95% confidence interval is  $p_1 - t_{\alpha/2,n-1}\sqrt{\frac{p_1 q_1}{n}}$ , or  $0.7071 - 1.9613\sqrt{\frac{0.7071(0.2929)}{1737}}$ , which is  $0.7071 - 0.0214$  or 0.6857
- And our upper bound is  $p_2 + t_{\alpha/2,n-1}\sqrt{\frac{p_2 q_2}{n}}$ , or  $0.7077 - 1.9613\sqrt{\frac{0.7077(0.2923)}{1737}}$ , which is  $0.7077 + 0.0214$ , or 0.7291

So the 95% SAIFS confidence interval for the population proportion,  $\pi$ , of Ebola Virus Disease patients with definite outcomes who will die is (0.686, 0.729).

## 29.8 A Function in R to Calculate the SAIFS Confidence Interval

I built an R function, called `saifs.ci` and contained in the Markdown for this document as well as the `R functions by T Love.R` script on the web site, which takes as its arguments a value for  $X$  = the number of successes,  $n$  = the number of trials, and `conf.level` = the confidence level, and produces the sample proportion, the SAIFS lower bound and upper bound for the specified two-sided confidence interval for the population proportion, using the equations above.

Here, for instance, are 95%, 90% and 99% confidence intervals for the population proportion  $\pi$  that we have been studying in the example about Ebola Virus disease.

```
saifs.ci(x = 1229, n = 1737)
```

Sample Proportion	0.025	0.975
0.708	0.686	0.729

```
saifs.ci(x = 1229, n = 1737, conf=0.9)
```

Sample Proportion	0.05	0.95
0.708	0.689	0.726

```
saifs.ci(x = 1229, n = 1737, conf=0.99, dig=5)
```

Sample Proportion	0.005	0.995
0.708	0.679	0.736

Note that in the final interval, I asked the machine to round to five digits rather than the default of three. On my desktop (and probably yours), doing so results in this output:

Sample Proportion	0.005	0.995
0.70754	0.67898	0.73585

I've got some setting wrong in my `bookdown` work so that this doesn't show up above when the function is called. Sorry!

### 29.8.1 The `saifs.ci` function in R

```
`saifs.ci` <-
  function(x, n, conf.level=0.95, dig=3)
  {
    p.sample <- round(x/n, digits=dig)

    p1 <- x / (n+1)
    p2 <- (x+1) / (n+1)

    var1 <- (p1*(1-p1))/n
    se1 <- sqrt(var1)
    var2 <- (p2*(1-p2))/n
    se2 <- sqrt(var2)

    lowq = (1 - conf.level)/2
    tcut <- qt(lowq, df=n-1, lower.tail=FALSE)

    lower.bound <- round(p1 - tcut*se1, digits=dig)
    upper.bound <- round(p2 + tcut*se2, digits=dig)
    res <- c(p.sample, lower.bound, upper.bound)
    names(res) <- c('Sample Proportion',lowq, 1-lowq)
    res
  }
```

## 29.9 Comparing the Confidence Intervals for the Ebola Virus Disease Example

Our three approaches give the following results:

Approach	95% CI for Population Proportion
<code>prop.test</code> (Wald)	(0.685, 0.729)
<code>binom.test</code> (Clopper & Pearson)	(0.686, 0.729)
<code>saifs.ci</code> (Borkowf)	(0.686, 0.729)

So in this case, it really doesn't matter which one you choose. With a smaller sample, we may not come to the same conclusion about the relative merits of these different approaches.

## 29.10 Can the Choice of Confidence Interval Method Matter?

The SAIFS approach will give a substantially different confidence interval than either the Wald or Clopper-Pearson approaches with a small sample size, and a probability of "success" that is close to either 0 or 1. For instance, suppose we run 10 trials, and obtain a single success, then use these data to estimate the true proportion of success,  $\pi$ .

The 95% confidence intervals under this circumstance are very different.

Method	R Command	95% CI for $\pi$
Wald	<code>prop.test(x = 1, n = 10)</code>	0.005, 0.459

Method	R Command	95% CI for $\pi$
Clopper-Pearson	<code>binom.test(x = 1, n = 10)</code>	0.003, 0.445
SAIFS	<code>saifs.ci(x = 1, n = 10)</code>	-0.115, 0.458

Note that the Wald and Clopper-Pearson procedures at least force the confidence interval to appear in the (0, 1) range. The SAIFS approach gives us some impossible values, and is thus a bit hard to take seriously – in reporting the result, we'd probably have to report the SAIFS interval as (0, 0.458).

If instead, we consider a situation where our null hypothesis is that the true proportion  $\pi$  is 0.10 (or 10%), and we run each of these three methods to obtain a 95% confidence interval, then we will come to somewhat different conclusions depending on the choice of method, if we observe 4 successes in 100 trials.

Method	R Command	95% CI for $\pi$
Wald	<code>prop.test(x = 4, n = 100)</code>	0.013, 0.105
Clopper-Pearson	<code>binom.test(x = 4, n = 100)</code>	0.011, 0.099
SAIFS	<code>saifs.ci(x = 4, n = 100)</code>	0.001, 0.093

Now, the Wald test suggests we retain the null hypothesis, the Clopper-Pearson test suggests we reject it (barely) and the SAIFS interval is more convinced that we should reject  $H_0 : \pi = 0.10$  in favor of  $H_A : \pi \neq 0.10$ .

**None of these three approaches is always better than the others.** When we have a sample size below 100, or the sample proportion of success is either below 0.10 or above 0.90, caution is warranted, although in many cases, the various methods give similar responses.

Data	Wald 95% CI	Clopper-Pearson 95% CI	SAIFS 95% CI
10 successes in 30 trials	0.179, 0.529	0.173, 0.528	0.148, 0.534
10 successes in 50 trials	0.105, 0.341	0.1, 0.337	0.083, 0.333
90 successes in 100 trials	0.82, 0.948	0.824, 0.951	0.829, 0.96
95 successes in 100 trials	0.882, 0.981	0.887, 0.984	0.894, 0.994

## Chapter 30

# Comparing Population Rates / Proportions

So, now we've built some methods for making statistical inferences about a single population proportion, the next step is to compare two proportions.

For instance, recall the Ebola Virus Disease study from the *New England Journal of Medicine*. Suppose we want to compare the proportion of deaths among cases that had a definitive outcome who were hospitalized to the proportion of deaths among cases that had a definitive outcome who were not hospitalized.

- We can summarize the data behind the two proportions we are comparing in a contingency table with two rows which identify the exposure or treatment of interest, and two columns to represent the outcomes of interest.
- In this case, we are comparing two groups of Ebola victims: those who were hospitalized and those who were not. The outcome of interest is whether the patient died or not.
- Our exposure is hospitalization and our outcome is death, and in the table we place the frequency for each combination of a row and a column.
- The rows need to be mutually exclusive and collectively exhaustive: each patient must either be hospitalized or not hospitalized. Similarly, the columns must meet the same standard: every patient is either dead or alive.

The article suggests that of the 1,737 cases with a definitive outcome, there were 1,153 hospitalized cases. Across those 1,153 hospitalized cases, 741 people (64.3%) died, which means that across the remaining 584 non-hospitalized cases, 488 people (83.6%) died.

Here is the initial contingency table, using only the numbers from the previous paragraph.

Initial Ebola Table	Deceased	Alive	Total
Hospitalized	741	—	1153
Not Hospitalized	488	—	584
Total			1737

Now, we can use arithmetic to complete the table, since the rows and the columns are each mutually exclusive and collectively exhaustive.

Ebola 2x2 Table	Deceased	Alive	Total
Hospitalized	741	412	1153
Not Hospitalized	488	96	584

Ebola 2x2 Table	Deceased	Alive	Total
Total	1229	508	1737

We want to compare the fatality risk (probability of being in the deceased column) for the population of people in the hospitalized row to the population of people in the not hospitalized row.

We do this by means of a hypothesis testing or confidence interval framework. The tricky part is that we have multiple ways to describe the relationship between hospitalization and death. We might compare the risks directly using the difference in probabilities, or the ratio of the two probabilities, or we might convert the risks to odds, and compare the ratio of those odds. In any case, we'll get slightly different  $p$  values and confidence intervals, all of which will help us answer the question about whether there is a statistically significant difference in fatality rates between those people who were hospitalized and those who were not. We'll return to this set of questions after discussing some of those approaches in a somewhat less depressing example.

## 30.1 Amoxicillin vs. Placebo for Otitis Media with Effusion

Van Balen et al. (1996) reported a double-blind placebo-controlled study of amoxicillin versus placebo for persistent otitis media with effusion (OME) in general practice<sup>1</sup>. The research question was whether antibiotic treatment is any better than watchful waiting. In this study, 162 children were randomized to receive amoxicillin or placebo. The outcome was the absence of persistent OME after two weeks of treatment.

## 30.2 The 2 by 2 Table

Data for the 149 children completing the two-week follow-up period are shown below:

Treatment Arm	Without OME	With OME	Total
Amoxicillin	37	42	79
Placebo	11	59	70
Total	48	101	149

This is an example of a 2x2 table, where we have the two treatments in the rows of the table, and the two possible outcomes in the columns of the table. This is an especially appropriate way to look at counts describing where subjects fall in the relationship between a categorical exposure/treatment and a categorical outcome.

## 30.3 Relating a Treatment to an Outcome

The question of interest is whether the percentage of amoxicillin kids without OME is different (specifically, larger) than the percentage of placebo kids without OME.

Treatment Arm	Without OME	With OME	Total	Proportion without OME
Amoxicillin	37	42	79	0.468

<sup>1</sup>Adapted from Woodworth GG (2004) Biostatistics: A Bayesian Introduction, Wiley. Table 7.1. page 109. Derived from van Balen et al. (1996). Lancet 348: 713-716, Table 4. Otitis media with effusion is occasionally referred to as "glue ear" and is the collection of effusion (fluid) that occurs within the middle-ear space due to the negative pressure produced by dysfunction of the Eustachian tube.

Treatment Arm	Without OME	With OME	Total	Proportion without OME
Placebo	11	59	70	0.157

In other words, what is the relationship between the treatment and the outcome?

### 30.4 Definitions of Probability and Odds

- Proportion = Probability = Risk of the trait = number with trait / total
- Odds of having the trait = (number with the trait / number without the trait) to 1

If  $p$  is the proportion of subjects with a trait, then the **odds** of having the trait are  $\frac{p}{1-p}$  to 1.

So, the probability of a good result (without OME) in this case is  $\frac{37}{79} = 0.4684$  in the amoxicillin group. The **odds** of a good result are thus  $\frac{0.4684}{1-0.4684} = 0.8811$  to 1.

Treatment	Without OME	With OME	Total	Pr(without OME)	Odds(without OME)
Amoxicillin	37	42	79	0.4684	0.8811
Placebo	11	59	70	0.1571	0.1864

### 30.5 Defining the Relative Risk

Among the amoxicillin subjects, the risk of a good outcome (without OME) is 46.84% or, stated as a proportion, 0.4684. Among the placebo subjects, the risk of a good outcome (without OME) is 15.71% or, stated as a proportion, 0.1571.

So our “crude” estimate of the **relative risk** of a good outcome for amoxicillin subjects as compared to placebo subjects, is the ratio of these two risks, or  $0.4684/0.1571 = 2.98$

- The fact that this relative risk is greater than 1 indicates that the probability of a good outcome is higher for amoxicillin subjects than for placebo subjects.
- A relative risk of 1 would indicate that the probability of a good outcome is the same for amoxicillin subjects and for placebo subjects.
- A relative risk less than 1 would indicate that the probability of a good outcome is lower for amoxicillin subjects than for placebo subjects.

### 30.6 Defining the Risk Difference

Our “crude” estimate of the **risk difference** of a good outcome for amoxicillin subjects as compared to placebo subjects, is  $0.4684 - 0.1571 = 0.3113$  or 31.1%

- The fact that this risk difference is greater than 0 indicates that the probability of a good outcome is higher for amoxicillin subjects than for placebo subjects.
- A risk difference of 0 would indicate that the probability of a good outcome is the same for amoxicillin subjects and for placebo subjects.
- A relative risk less than 0 would indicate that the probability of a good outcome is lower for amoxicillin subjects than for placebo subjects.

## 30.7 Defining the Odds Ratio, or the Cross-Product Ratio

Among the amoxicillin subjects, the odds of a good outcome (without OME) are 0.8811. Among the placebo subjects, the odds of a good outcome (without OME) are .1864.

So our “crude” estimate of the **odds ratio** of a good outcome for amoxicillin subjects as compared to placebo subjects, is  $0.8811 / 0.1864 = 4.73$

Another way to calculate this odds ratio is to calculate the **cross-product ratio**, which is equal to  $(ad) / (bc)$ , for the 2 by 2 table with counts specified as shown:

A Generic Table	Good Outcome	Bad Outcome
Treatment Group 1	a	b
Treatment Group 2	c	d

So, for our table, we have  $a = 37$ ,  $b = 42$ ,  $c = 11$ , and  $d = 59$ , so the cross-product ratio is  $\frac{37 \times 59}{42 \times 11} = \frac{2183}{462} = 4.73$ . As expected, this is the same as the “crude” odds ratio estimate.

- The fact that this odds ratio risk is greater than 1 indicates that the odds of a good outcome are higher for amoxicillin subjects than for placebo subjects.
- An odds ratio of 1 would indicate that the odds of a good outcome are the same for amoxicillin subjects and for placebo subjects.
- An odds ratio less than 1 would indicate that the odds of a good outcome are lower for amoxicillin subjects than for placebo subjects.

So, we have several different ways to compare the outcomes across the treatments. Are these differences and ratios large enough to rule out chance?

## 30.8 Comparing Rates in a 2x2 Table

The key question is whether the percentage of amoxicillin kids without OME is statistically significantly different (specifically, larger) than the percentage of placebo kids without OME. In other words, what is the relationship between the treatment and the outcome in the following two-by-two table?

Treatment Arm	(Good Outcome) Without OME	(Bad Outcome) With OME	Total
Amoxicillin	37	42	79
Placebo	11	59	70
<b>Total</b>	<b>48</b>	<b>101</b>	<b>149</b>

## 30.9 The twobytwo function in R

I built the **twobytwo** function in R (based on existing functions in the **Epi** library, which you need to have in your available Packages list in order for this to work) to do the work for this problem. All that is required is a single command, and a two-by-two table like this one, in standard epidemiological format (with the outcomes in the columns, and the treatments in the rows.)

The command just requires you to read off the cells of the table, followed by the labels for the two treatments, then the two outcomes, in this order:

```
twobytwo(37,42,11,59, "Amoxicillin", "Placebo", "Good", "Bad")
```

The resulting output follows. We'll walk through it all in a moment.

```
twobytwo(37, 42, 11, 59, "Amoxicillin", "Placebo", "Good", "Bad")
```

2 by 2 table analysis:

---

Outcome : Good

Comparing : Amoxicillin vs. Placebo

	Good	Bad	P(Good)	95% conf. interval
Amoxicillin	37	42	0.468	0.3615 0.578
Placebo	11	59	0.157	0.0892 0.262

95% conf. interval

Relative Risk: 2.980 1.650 5.383

Sample Odds Ratio: 4.725 2.164 10.316

Conditional MLE Odds Ratio: 4.675 2.051 11.384

Probability difference: 0.311 0.164 0.439

Exact P-value: 0

Asymptotic P-value: 1e-04

---

The main conclusion for the data using any of these tests and confidence intervals, is that with 95% confidence, we can conclude that the probability of a good outcome (i.e. no OME at two weeks) is significantly higher with the use of Amoxicillin as compared to placebo.

## 30.10 Walking through the twobytwo function's Results

### 30.10.1 Outcome Probabilities and Confidence Intervals Within the Treatment Groups

The output starts with estimates of the probability (risk) of a Good Outcome among patients who fall into the two treatment groups (Amoxicillin or Placebo), along with 95% confidence intervals for each of these probabilities.

2 by 2 table analysis:

---

Outcome : Good

Comparing : Amoxicillin vs. Placebo

	Good	Bad	P(Good)	95% conf. interval
Amoxicillin	37	42	0.4684	0.3615 0.5781
Placebo	11	59	0.1571	0.0892 0.2619

The conditional probability of a Good outcome given that the patient is in the Amoxicillin treatment arm, is symbolized as  $\text{Pr}(\text{Good} | \text{Amoxicillin}) = 0.4684$ .

- Note that if these two confidence intervals fail to overlap (as these do) then we would expect to find a statistically significant difference in probability of a good outcome when we compare amoxicillin to placebo.
- If the two confidence intervals overlap, then we don't know whether the difference will be statistically significant or not yet.

### 30.10.2 Relative Risk, Odds Ratio and Risk Difference, with Confidence Intervals

These elements are followed by estimates of the relative risk, odds ratio, and risk difference, each with associated 95% confidence intervals.

	95% conf. interval		
Relative Risk:	2.9804	1.6501	5.3832
Sample Odds Ratio:	4.7251	2.1643	10.3157
Conditional MLE Odds Ratio:	4.6746	2.0509	11.3837
Probability difference:	0.3112	0.1636	0.4391

- The **relative risk**, or the ratio of  $P(\text{Good Outcome} | \text{Amoxicillin})$  to  $P(\text{Good Outcome} | \text{Placebo})$ , is shown first. Note that the 95% confidence interval is entirely greater than 1, suggesting that the true relative risk is significantly greater than 1, and thus that the probability of a good outcome is significantly more likely for amoxicillin.
- The **odds ratio** is presented using two different definitions (the sample odds ratio is the cross-product ratio we mentioned earlier). Note that the 95% confidence interval using either approach is entirely greater than 1, suggesting that the true odds ratio is significantly greater than 1, and thus that the odds and thus also the probability of a good outcome is significantly more likely for amoxicillin.
- The **probability (or risk) difference** [ $P(\text{Good Outcome} | \text{Amoxicillin}) - P(\text{Good Outcome} | \text{Placebo})$ ] is presented last. Note that the 95% confidence interval is entirely greater than 0, suggesting that the true risk difference is significantly greater than 0, and thus that the probability of a good outcome is significantly more likely for amoxicillin.
- Note carefully that if there had been no difference between Amoxicillin and Placebo, the relative risk and odds ratios would be 1, but the probability difference would be zero.

### 30.10.3 Hypothesis Testing Results

Finally, the output gives  $p$  values for both a Fisher's exact test (exact) and Pearson  $\chi^2$  test (asymptotic) of the hypotheses

- $H_0$ : Rows and Columns are statistically independent. vs.
- $H_A$ : Rows and Columns are associated with (or dependent on) each other.

Exact P-value: 0  
Asymptotic P-value: 1e-04

Here, the tiny  $p$  values (in both cases,  $p < 0.001$ ) suggest that we should reject  $H_0$  and conclude that the outcome probabilities are associated with the treatment received. In other words, which Treatment group you're in significantly affects the probability of obtaining a Good outcome, which is the same conclusion we've drawn from each of the confidence intervals presented above.

## 30.11 Estimating a Rate More Accurately: Use $(x + 1)/(n + 2)$ rather than $x/n$

Suppose you have some data involving  $n$  independent tries, with  $x$  successes. A natural estimate of the “success rate” in the data is  $x / n$ .

But, strangely enough, it turns out this isn't an entirely satisfying estimator. Alan Agresti provides substantial motivation for the  $(x + 1)/(n + 2)$  estimate as an alternative<sup>2</sup>. This is sometimes called a *Bayesian augmentation*.

---

<sup>2</sup>This note comes largely from a May 15 2007 entry in Andrew Gelman's blog at <http://andrewgelman.com/2007/05/15>

- The big problem with  $x / n$  is that it estimates  $p = 0$  or  $p = 1$  when  $x = 0$  or  $x = n$ .
- It's also tricky to compute confidence intervals at these extremes, since the usual standard error for a proportion,  $\sqrt{np(1-p)}$ , gives zero, which isn't quite right.
- $(x + 1)/(n + 2)$  is much cleaner, especially when you build a confidence interval for the rate.
- The only place where  $(x + 1)/(n + 2)$  will go wrong (as in the SAIFS approach) is if  $n$  is small and the true probability is very close to 0 or 1.

For example, if  $n = 10$ , and  $p$  is 1 in a million, then  $x$  will almost certainly be zero, and an estimate of  $1/12$  is much worse than the simple  $0/10$ . However, how big a deal is this? If  $p$  might be 1 in a million, you're not going to estimate it with a  $n = 10$  experiment<sup>3</sup>.

## 30.12 Back to the OTE example

Returning to our example, let's run an augmented analysis (with one extra "Good" and one extra "Bad" in each of the treatment groups)

```
twobytwo(37+1, 42+1, 11+1, 59+1, "Amoxicillin", "Placebo", "Good", "Bad")
```

2 by 2 table analysis:

```
-----  
Outcome : Good  
Comparing : Amoxicillin vs. Placebo  
  
          Good Bad    P(Good) 95% conf. interval  
Amoxicillin   38  43      0.469    0.3635    0.578  
Placebo        12  60      0.167    0.0972    0.271  
  
                           95% conf. interval  
Relative Risk: 2.815      1.598     4.96  
Sample Odds Ratio: 4.419      2.071     9.43  
Conditional MLE Odds Ratio: 4.375      1.965    10.33  
Probability difference: 0.302      0.156     0.43  
  
Exact P-value: 1e-04  
Asymptotic P-value: 1e-04
```

Note that the augmentation moves both the estimate and interval endpoints towards 0.50.

## 30.13 Does the Bayesian Augmentation $(x + 1)/(n + 2)$ Matter, Practically?

Generally, this augmentation doesn't matter much at all in any setting where you have a reasonably large sample size, or where the sample probability of success in each group isn't too close to 0 or 1.

Suppose you have 50 subjects who were exposed to some stimulus, and another 45 who were not. Of the 50 exposed subjects, 20 have the outcome of interest, while this is true for 9 of the unexposed subjects. What conclusions do we draw, first without and then with this Bayesian augmentation?

First, without the augmentation:

---

<sup>3</sup> Andrew Gelman's example is "I'm not going to try ten 100-foot golf putts, miss all of them, and then estimate my probability of success as  $1/12$ ."

```
twobytwo(20,30,9,36, "Exposed", "Not Exposed", "Has Outcome", "No Outcome")
```

2 by 2 table analysis:

Outcome : Has Outcome  
Comparing : Exposed vs. Not Exposed

	Has Outcome	No Outcome	P(Has Outcome)	95% conf. interval
Exposed	20	30	0.4	0.275 0.540
Not Exposed	9	36	0.2	0.107 0.342
<hr/>				
			95% conf. interval	
Relative Risk:	2.00	1.0175	3.931	
Sample Odds Ratio:	2.67	1.0585	6.718	
Conditional MLE Odds Ratio:	2.64	0.9746	7.629	
Probability difference:	0.20	0.0144	0.365	
<hr/>				
			Exact P-value:	0.0451
			Asymptotic P-value:	0.0375
<hr/>				

And now, with the augmentation:

```
twobytwo(21,31,10,37, "Exposed", "Not Exposed", "Has Outcome", "No Outcome")
```

2 by 2 table analysis:

Outcome : Has Outcome  
Comparing : Exposed vs. Not Exposed

	Has Outcome	No Outcome	P(Has Outcome)	95% conf. interval
Exposed	21	31	0.404	0.280 0.541
Not Exposed	10	37	0.213	0.118 0.352
<hr/>				
			95% conf. interval	
Relative Risk:	1.898	0.999	3.605	
Sample Odds Ratio:	2.506	1.028	6.113	
Conditional MLE Odds Ratio:	2.483	0.950	6.859	
Probability difference:	0.191	0.008	0.355	
<hr/>				
			Exact P-value:	0.0517
			Asymptotic P-value:	0.0434
<hr/>				

It is likely that the augmented version is a more accurate estimate here, but the two estimates will be comparable, generally, so long as either (a) the sample size in each exposure group is more than, say, 30 subjects, and/or (b) the sample probability of the outcome is between 10% and 90% in each exposure group.

## 30.14 Hypothesis Testing About a Population Proportion

To perform a hypothesis test about a population proportion, we'll usually use the `prop.test` or `binom.test` approaches in R.

- The null hypothesis is that the population proportion is equal to some pre-specified value. Often, this

is taken to be 0.5, but it can be any value, called  $\pi_0$  that is between 0 and 1.

- The alternative hypothesis may be one-sided or two-sided. If it is two-sided, it will be that the population proportion is not equal to the value  $\pi_0$  specified by the null hypothesis.
- In the two-sided case, we have  $H_0 : \pi = \pi_0$  and  $H_A : \pi \neq \pi_0$
- In the one-sided “greater than” case, we have  $H_0 : \pi \leq \pi_0$  and  $H_A : \pi > \pi_0$

As an example, suppose we want to see if the evidence available so far is enough to conclude that the population case fatality rate across the countries included in the WHO’s report is more than 67% (i.e. more than two-thirds of those with definitive outcomes will die), and we want to do this using a 5% significance level.

We could use `prop.test` or `binom.test` here.

```
binom.test(x = 1229, n = 1737, p = 0.67, alternative="greater")
```

Exact binomial test

```
data: 1229 and 1737
number of successes = 1000, number of trials = 2000, p-value =
4e-04
alternative hypothesis: true probability of success is greater than 0.67
95 percent confidence interval:
0.689 1.000
sample estimates:
probability of success
0.708
prop.test(x = 1229, n = 1737, p = 0.67, alternative="greater")
```

1-sample proportions test with continuity correction

```
data: 1229 out of 1737, null probability 0.67
X-squared = 10, df = 1, p-value = 5e-04
alternative hypothesis: true p is greater than 0.67
95 percent confidence interval:
0.689 1.000
sample estimates:
p
0.708
```

1. What conclusion should we draw here?
2. Does it matter which of the two test procedures we use?
3. Do the  $p$  values match up with the 95% confidence intervals?

## 30.15 Assumptions for Inferences about a Population Proportion

1. There are  $n$  identical trials.
2. There are two possible outcomes (designated as success and failure) for each trial.
3. The true probability of success,  $\pi$ , remains constant across trials.
4. Each trial is independent of all of the other trials.

In order for the confidence intervals and tests we produce to remain reasonably accurate, we’d also like to see that both  $np =$  the observed number of successes and  $n(1-p) =$  the observed number of failures are greater

than 5. If not, then the intervals may be incorrect (shifted away from the true value of  $\pi$ ), and also less efficient (wider) than necessary.

## 30.16 Building a 2x2 Table in R from a Data Frame

Remember our first-day survey? It's in the `surveyday1.csv` file on our website, and loaded as the `survey1` data frame here. Two of the questions on that survey asked you to specify your sex and whether English was your first language. Do men and women have statistically significantly different probabilities of being native speakers of English?

```
table(survey1$sex, survey1$english)
```

	n	y
f	13	57
m	16	68

I would like to make those a little easier to read, so I'm going to change the labels for the levels without changing their order.

```
survey1$sex.new <- factor(survey1$sex, labels = c("Female", "Male"))
survey1$lang1 <- factor(survey1$english, labels = c("Not English", "English"))

table(survey1$sex.new, survey1$lang1)
```

	Not English	English
Female	13	57
Male	16	68

## 30.17 Standard Epidemiological Format

Now, suppose we want this in **standard epidemiological format**, which means that:

- The rows of the table describe the “treatment” (which we'll take here to be sex). The more interesting (sometimes also the more common) “treatment” is placed in the top row.
- The columns of the table describe the “outcome” (which we'll take here to be whether English was your first language.) Typically, the more common “outcome” is placed to the left.

So, for standard format, we want to get the “Female” and “English” cell to the top left of the table, not the “Female” and “Not English” cell that is there now.

So, we are going to reorder the `english` variable's levels to accomplish this:

```
survey1$lang.new <- factor(survey1$lang1, levels=c("English", "Not English"))
table(survey1$sex.new, survey1$lang.new)
```

	English	Not English
Female	57	13
Male	68	16

And now, we can seamlessly grab these results and insert them into the `twoby2` function from the `Epi` package...

```
twoby2(survey1$sex.new, survey1$lang.new)
```

2 by 2 table analysis:

Outcome : English  
Comparing : Female vs. Male

	English	Not English	P(English)	95% conf. interval
Female	57	13	0.814	0.706 0.889
Male	68	16	0.809	0.711 0.880
<hr/>				
			95% conf. interval	
Relative Risk:	1.0059		0.864	1.172
Sample Odds Ratio:	1.0317		0.458	2.324
Conditional MLE Odds Ratio:	1.0315		0.424	2.545
Probability difference:	0.0048		-0.122	0.126
<hr/>				
			Exact P-value:	1
			Asymptotic P-value:	0.94
<hr/>				

## 30.18 Use the Bayesian Augmentation $(x + 1)/(n + 2)$

As a default estimate for a rate,  $(x + 1)/(n + 2)$  is a better choice than  $x / n$ . Add a success and a failure to your data to get a better estimate (especially a confidence interval) of a population rate. This is sometimes called a Bayesian augmentation of the data. Occasionally, statisticians will use a more extensive adjustment, like  $(x + 2) / (n + 4)$ , even.

If we like, we can analyze the sex-language relationship including the Bayesian augmentation<sup>4</sup>:

```
twoby2(table(survey1$sex.new, survey1$lang.new) + 1)
```

2 by 2 table analysis:

Outcome : English  
Comparing : Female vs. Male

	English	Not English	P(English)	95% conf. interval
Female	58	14	0.806	0.698 0.881
Male	69	17	0.802	0.705 0.873
<hr/>				
			95% conf. interval	
Relative Risk:	1.0040		0.860	1.172
Sample Odds Ratio:	1.0207		0.464	2.246
Conditional MLE Odds Ratio:	1.0206		0.431	2.446
Probability difference:	0.0032		-0.124	0.125
<hr/>				
			Exact P-value:	1
			Asymptotic P-value:	0.959
<hr/>				

<sup>4</sup>Note that simply adding one to each cell in the table is what we are looking for.

### 30.19 Returning to the Ebola Virus Disease Survival Example

Recall our 2x2 table comparing case fatality rates by whether the subject was hospitalized.

Ebola 2x2 Table	Deceased	Alive	Total
Hospitalized	741	412	1153
Not Hospitalized	488	96	584
Total	1229	508	1737

We can run these data through R, using the augmentation (adding a death and a survival to the hospitalized and also to the not hospitalized groups.)

```
twobytwo(741+1, 412+1, 488+1, 96+1,
         "Hospitalized", "Not Hospitalized", "Deceased", "Alive")
```

2 by 2 table analysis:

```
-----
Outcome : Deceased
Comparing : Hospitalized vs. Not Hospitalized

          Deceased Alive   P(Deceased) 95% conf. interval
Hospitalized        742    413      0.642      0.614      0.670
Not Hospitalized    489     97      0.835      0.802      0.862

          95% conf. interval
Relative Risk: 0.770      0.728      0.814
Sample Odds Ratio: 0.356      0.278      0.457
Conditional MLE Odds Ratio: 0.357      0.275      0.460
Probability difference: -0.192     -0.232     -0.150

          Exact P-value: 0
          Asymptotic P-value: 0
-----
```

- What conclusions can you draw from the R output above?

Now, in the same *New England Journal of Medicine* article, data are provided for the percentage of deaths among male and female patients, for a slightly different group of EVD patients. In that group, there were 874 men, of whom 631 died, and 818 women, of whom 572 died.

- Specify the null and alternative hypotheses that can be tested for these new data.
- Develop the appropriate 2x2 table and get it into R for analysis.
- What conclusions can we draw from your comparison of fatality risks by sex?

#### 30.19.1 Answer Sketch for questions 2-4

The null hypothesis is that the population death rate among men is the same as the population death rate among women, against a two-sided alternative (that the rates are not the same)

Here is the appropriate set of 2x2 table results, including the Bayesian augmentation.

```
twobytwo(631 + 1, (874 - 631) + 1, 572 + 1, (818 - 572) + 1,
         "Men", "Women", "Died", "Survived")
```

2 by 2 table analysis:

---

Outcome : Died  
Comparing : Men vs. Women

	Died	Survived	P(Died)	95% conf. interval
Men	632	244	0.722	0.691 0.750
Women	573	247	0.699	0.666 0.729
<hr/>				
				95% conf. interval
Relative Risk:	1.0325		0.9714	1.0973
Sample Odds Ratio:	1.1165		0.9051	1.3774
Conditional MLE Odds Ratio:	1.1165		0.9000	1.3851
Probability difference:	0.0227		-0.0205	0.0658
<hr/>				
			Exact P-value:	0.309
			Asymptotic P-value:	0.303

---

Our conclusions are (from any of the comparisons) that the survival rates do not differ significantly by sex, at least at the 5% significance level. We could use the relative risk, odds ratio, probability difference, or even chi-square test, to see this.



# Chapter 31

## Power and Sample Size for Comparing Two Population Proportions

### 31.1 Tuberculosis Prevalence Among IV Drug Users

Pagano and Gauvreau (2000) describe a study to investigate factors affecting tuberculosis prevalence among intravenous drug users<sup>1</sup>. Among 97 individuals who admit to sharing needles, 24 (24.7%) had a positive tuberculin skin test result; among 161 drug users who deny sharing needles, 28 (17.4%) had a positive test result. To start, we'll test the null hypothesis that the proportions of intravenous drug users who have a positive tuberculin skin test result are identical for those who share needles and those who do not.

```
twobytwo(24, 73, 28, 133,  
         "Sharing Needles", "Not Sharing",  
         "TB test+", "TB test-")
```

2 by 2 table analysis:

```
-----  
Outcome : TB test+  
Comparing : Sharing Needles vs. Not Sharing
```

	TB test+	TB test-	P(TB test+)	95% conf. interval
Sharing Needles	24	73	0.247	0.172 0.343
Not Sharing	28	133	0.174	0.123 0.240

	95% conf. interval	
Relative Risk:	1.4227	0.8772 2.307
Sample Odds Ratio:	1.5616	0.8439 2.890
Conditional MLE Odds Ratio:	1.5588	0.8014 3.019
Probability difference:	0.0735	-0.0265 0.181

	Exact P-value:	Asymptotic P-value:
	0.2	0.156

```
-----
```

What conclusions should we draw?

---

<sup>1</sup>Original Data Source: Graham NMH et al. (1992) Prevalence of Tuberculin Positivity and Skin Test Anergy in HIV-1-Seropositive and Seronegative Intravenous Drug Users. JAMA, 267, 369-373.

## 31.2 Designing a New TB Study

Now, suppose we wanted to design a new study with as many non-sharers as needle-sharers participating, and suppose that we wanted to detect any difference in the proportion of positive skin test results between the two groups that was identical to the data presented above or larger with at least 90% power, using a two-sided test and  $\alpha = .05$ . What sample size would be required to accomplish these aims?

## 31.3 Using `power.prop.test` for Balanced Designs

Our constraints are that we want to find the sample size for a two-sample comparison of proportions using a balanced design, we will use  $\alpha = .05$ , and power = .90, and that we estimate that the non-sharers will have a .174 proportion of positive tests, and we will try to detect a difference between this group and the needle sharers, who we estimate will have a proportion of .247, using a two-sided hypothesis test.

```
power.prop.test(p1 = .174, p2 = .247, sig.level = 0.05, power = 0.90)
```

Two-sample comparison of proportions power calculation

```
n = 653
p1 = 0.174
p2 = 0.247
sig.level = 0.05
power = 0.9
alternative = two.sided
```

NOTE: n is number in \*each\* group

So, we'd need at least 654 non-sharing subjects, and 654 more who share needles to accomplish the aims of the study.

## 31.4 How `power.prop.test` works

`power.prop.test` works much like the `power.t.test` we saw for means.

Again, we specify 4 of the following 5 elements of the comparison, and R calculates the fifth.

- The sample size (interpreted as the # in each group, so half the total sample size)
- The true probability in group 1
- The true probability in group 2
- The significance level ( $\alpha$ )
- The power ( $1 - \beta$ )

The big weakness with the `power.prop.test` tool is that it doesn't allow you to work with unbalanced designs.

## 31.5 Another Scenario

Suppose we can get exactly 800 subjects in total (400 sharing and 400 non-sharing). How much power would we have to detect a difference in the proportion of positive skin test results between the two groups that was identical to the data presented above or larger, using a one-sided test, with  $\alpha = .10$ ?

```
power.prop.test(n=400, p1=.174, p2=.247, sig.level = 0.10,
                alternative="one.sided")
```

Two-sample comparison of proportions power calculation

```
n = 400
p1 = 0.174
p2 = 0.247
sig.level = 0.1
power = 0.895
alternative = one.sided
```

NOTE: n is number in \*each\* group

We would have just under 90% power to detect such an effect.

## 31.6 Using the pwr library to assess sample size for Unbalanced Designs

The `pwr.2p2n.test` function in the `pwr` library can help assess the power of a test to determine a particular effect size using an unbalanced design, where  $n_1$  is not equal to  $n_2$ .

As before, we specify four of the following five elements of the comparison, and R calculates the fifth.

- `n1` = The sample size in group 1
- `n2` = The sample size in group 2
- `sig.level` = The significance level ( $\alpha$ )
- `power` = The power ( $1 - \beta$ )
- `h` = the effect size  $h$ , which can be calculated separately in R based on the two proportions being compared: `p1` and `p2`.

### 31.6.1 Calculating the Effect Size `h`

To calculate the effect size for a given set of proportions, just use `ES.h(p1, p2)` which is available in the `pwr` library.

For instance, in our comparison, we have the following effect size.

```
ES.h(p1 = .174, p2 = .247)
```

```
[1] -0.18
```

## 31.7 Using `pwr.2p2n.test` in R

Suppose we can have 700 samples in group 1 (the not sharing group) but only half that many in group 2 (the group of users who share needles). How much power would we have to detect this same difference ( $p_1 = .174$ ,  $p_2 = .247$ ) with a 5% significance level in a two-sided test?

```
pwr.2p2n.test(h = ES.h(p1 = .174, p2 = .247),
               n1 = 700, n2 = 350,
               sig.level = 0.05)
```

```
difference of proportion power calculation for binomial distribution (arcsine transformation)
```

```
h = 0.18
n1 = 700
n2 = 350
sig.level = 0.05
power = 0.784
alternative = two.sided
```

NOTE: different sample sizes

Note that the headline for this output actually reads:

```
difference of proportion power calculation for binomial distribution
(arcsine transformation)
```

It appears we will have about 78% power under these circumstances.

### 31.7.1 Comparison to Balanced Design

How does this compare to the results with a balanced design using only 1000 drug users in total, so that we have 500 patients in each group?

```
pwr.2p2n.test(h = ES.h(p1 = .174, p2 = .247), n1 = 500, n2 = 500, sig.level = 0.05)
```

```
difference of proportion power calculation for binomial distribution (arcsine transformation)
```

```
h = 0.18
n1 = 500
n2 = 500
sig.level = 0.05
power = 0.811
alternative = two.sided
```

NOTE: different sample sizes

or we could instead have used...

```
power.prop.test(p1 = .174, p2 = .247, sig.level = 0.05, n = 500)
```

Two-sample comparison of proportions power calculation

```
n = 500
p1 = 0.174
p2 = 0.247
sig.level = 0.05
power = 0.809
alternative = two.sided
```

NOTE: n is number in \*each\* group

Note that these two sample size estimation approaches are approximations, and use slightly different approaches, so it's not surprising that the answers are similar, but not completely identical.

# Chapter 32

## Larger Contingency Tables - Testing for Independence

What will we do with tables describing data from more than two categories at a time, returning to the notion of independent (rather than paired or matched) samples? The chi-square tests we have already seen in our `twobytwo` table output will extend nicely to this scenario, especially the Pearson  $\chi^2$  (asymptotic) test.

### 32.1 A 2x3 Table: Comparing Response to Active vs. Placebo

The table below, containing 2 rows and 3 columns of data (ignoring the marginal totals) specifies the number of patients who show *complete*, *partial*, or *no response* after treatment with either **active** medication or a **placebo**.

Group	None	Partial	Complete
Active	16	26	29
Placebo	24	26	18

Is there a statistically significant association here? That is to say, is there a statistically significant difference between the treatment groups in the distribution of responses?

#### 32.1.1 Getting the Table into R

To answer this, we'll have to get the data from this contingency table into a matrix in R. Here's one approach...

```
T1 <- matrix(c(16, 26, 29, 24, 26, 18), ncol=3, nrow=2, byrow=TRUE)
rownames(T1) <- c("Active", "Placebo")
colnames(T1) <- c("None", "Partial", "Complete")

T1
```

	None	Partial	Complete
Active	16	26	29
Placebo	24	26	18

### 32.1.2 Manipulating the Table's presentation

We can add margins to the matrix to get a table including row and column totals.

```
addmargins(T1)
```

	None	Partial	Complete	Sum
Active	16	26	29	71
Placebo	24	26	18	68
Sum	40	52	47	139

Instead of the counts, we can tabulate the proportion of all patients within each cell.

```
prop.table(T1)
```

	None	Partial	Complete
Active	0.115	0.187	0.209
Placebo	0.173	0.187	0.129

Or, we can tabulate the probabilities, rather than the proportions, after some rounding.

```
round(100*prop.table(T1), 1)
```

	None	Partial	Complete
Active	11.5	18.7	20.9
Placebo	17.3	18.7	12.9

R can also plot the percentages conditional on the rows...

```
round(100*prop.table(T1, 1), 1)
```

	None	Partial	Complete
Active	22.5	36.6	40.8
Placebo	35.3	38.2	26.5

The 40.8 in Active/Complete means that of the Active medication patients, 40.8% had a complete response.

R can also plot the percentages conditional on the columns...

```
round(100*prop.table(T1, 2), 1)
```

	None	Partial	Complete
Active	40	50	61.7
Placebo	60	50	38.3

If we add the row of column totals for these percentages as shown below, it clarifies that the 61.7 in Active/Complete here means that of the patients with a Complete response, 61.7% were on the active medication.

```
addmargins(round(100*prop.table(T1, 2), 1), 1)
```

	None	Partial	Complete
Active	40	50	61.7
Placebo	60	50	38.3
Sum	100	100	100.0

## 32.2 Getting the Chi-Square Test Results

Now, to actually obtain a  $p$  value and perform the significance test with  $H_0$ : rows and columns are independent vs.  $H_A$ : rows and columns are associated, we simply run a Pearson chi-square test on T1 ...

```
chisq.test(T1)
```

Pearson's Chi-squared test

```
data: T1
X-squared = 4, df = 2, p-value = 0.1
```

Thanks to a p-value of about 0.13 (using the Pearson  $\chi^2$  test) our conclusion would be to retain the null hypothesis of independence in this setting.

We could have run a Fisher's exact test, too, if we needed it.

```
fisher.test(T1)
```

Fisher's Exact Test for Count Data

```
data: T1
p-value = 0.1
alternative hypothesis: two.sided
rm(T1)
```

The Fisher exact test  $p$  value is also 0.13. Either way, there is insufficient evidence to conclude that there is a (true) difference in the distributions of responses.

### 32.3 Getting a 2x3 Table into R using a .csv file

Suppose we have a table like this available, and want to compute the Pearson and Fisher  $\chi^2$  test results in R, without having to set up the whole matrix piece discussed above?

Group	None	Partial	Complete
Active	16	26	29
Placebo	24	26	18

We could do so by building a .csv file (a spreadsheet) containing the table above. In particular, I built a file called `active2x3.csv`, available on the course website. It is simply the table below.

Group	None	Partial	Complete
Active	16	26	29
Placebo	24	26	18

When we pull this .csv file into R, it emerges as a data frame and deliberately NOT as a tibble.

```
active2x3
```

	Group	None	Partial	Complete
1	Active	16	26	29
2	Placebo	24	26	18

## 32.4 Turning the Data Frame into a Table That R Recognizes

We need to turn this data frame into something that R can recognize as a contingency table. The steps to do this are:

1. Establish row names from the Group variable.
2. Delete the Group variable, retaining only the variables containing counts.
3. Convert the data frame into a matrix.

Specifically in this case, the steps are:

```
rownames(active2x3) <- active2x3$Group
active2x3$Group <- NULL
tab2x3 <- as.matrix(active2x3)
tab2x3
```

	None	Partial	Complete
Active	16	26	29
Placebo	24	26	18

And this resulting `tab2x3` object can be treated as the matrix was previously, using `addmargins` or `chisq.test` or `prop.table` or whatever. For instance,

```
fisher.test(tab2x3)
```

Fisher's Exact Test for Count Data

```
data: tab2x3
p-value = 0.1
alternative hypothesis: two.sided
```

## 32.5 Collapsing Levels / Categories in a Factor

Returning to the `survey1` data, let's build a table of the relationship between response to the `sex` and favorite color questions.

```
table(survey1$sex, survey1$color)
```

	aqua	aquamarine	black	blue	brown	chartreuse	dark blue	depends	gray	
f	2	0	1	21	0	0	2	0	0	
m	0	1	2	41	3	1	0	1	1	
	green	grey	light blue	navy	none	orange	pink	purple	red	royal blue
f	11	1	1	0	1	0	4	10	5	1
m	12	2	0	2	0	3	1	1	8	0
	sapphire	silver	teal	violet	white	yellow				
f	1	1	1	1	3	3				
m	0	0	0	0	2	1				

```
chisq.test(table(survey1$sex, survey1$color))
```

Warning in chisq.test(table(survey1\$sex, survey1\$color)): Chi-squared approximation may be incorrect

### Pearson's Chi-squared test

```
data: table(survey1$sex, survey1$color)
X-squared = 40, df = 20, p-value = 0.02
```

Note the warning message. With all those small cell counts, and particular, so many zeros, this might not be so useful.

We might reasonably consider collapsing the `colors` data into four new categories:

- Blue – in which I will include the old aqua, aquamarine, blue, dark blue and sapphire;
- Green, which will include the old chartreuse and green,
- Red - which will include the old orange, pink and red, and
- Other – which will include all other colors, including purple and violet

One way to do this sort of work uses the `plyr` library in R, and the `revalue` function in particular. I learned about this in Chapter 15 of Chung's R Graphics Cookbook, which is invaluable.

```
survey1$color.new <- factor(survey1$color)
levels(survey1$color.new)

[1] "aqua"      "aquamarine" "black"       "blue"        "brown"
[6] "chartreuse" "dark blue"   "depends"     "gray"        "green"
[11] "grey"       "light blue"  "navy"        "none"        "orange"
[16] "pink"       "purple"     "red"         "royal blue"  "sapphire"
[21] "silver"     "teal"       "violet"      "white"       "yellow"

survey1$color.new <- dplyr::recode(survey1$color.new,
  aqua = "Blue", aquamarine="Blue", black = "Other",
  blue = "Blue", brown = "Other", chartreuse = "Green",
  'dark blue' = "Blue", depends = "Other", gray = "Other",
  green = "Green", grey ="Other", 'light blue' = "Blue",
  navy = "Blue", none = "Other", orange = "Red",
  pink = "Red", purple = "Other", red = "Red",
  'royal blue' = "Blue", sapphire = "Blue", silver = "Other",
  teal = "Blue", violet = "Other", white = "Other", yellow = "Other")
```

So, what I've done here is create a new color variable that assigns the original colors into the four categories: Blue, Green, Red and Other that I defined above. Let's run a sanity check on our recoding, then look at the relationship between sex and this new four-category variable, in a 2x4 table...

```
table(survey1$color, survey1$color.new) ## sanity check
```

	Blue	Other	Green	Red
aqua	2	0	0	0
aquamarine	1	0	0	0
black	0	3	0	0
blue	62	0	0	0
brown	0	3	0	0
chartreuse	0	0	1	0
dark blue	2	0	0	0
depends	0	1	0	0
gray	0	1	0	0
green	0	0	23	0
grey	0	3	0	0
light blue	1	0	0	0

navy	2	0	0	0
none	0	1	0	0
orange	0	0	0	3
pink	0	0	0	5
purple	0	11	0	0
red	0	0	0	13
royal blue	1	0	0	0
sapphire	1	0	0	0
silver	0	1	0	0
teal	1	0	0	0
violet	0	1	0	0
white	0	5	0	0
yellow	0	4	0	0

```
table(survey1$sex, survey1$color.new)
```

	Blue	Other	Green	Red
f	29	21	11	9
m	44	13	13	12

To neaten this output up, we might want to have Other show up last in the `color.new` group.

```
survey1$color.new2 <- factor(survey1$color.new,
    levels=c("Blue", "Green", "Red", "Other"))
table(survey1$sex, survey1$color.new2)
```

	Blue	Green	Red	Other
f	29	11	9	21
m	44	13	12	13

Now, we run the new Pearson  $\chi^2$  test, and conclude that there is no evidence of statistically significant association (at the 5% significance level) between sex and these collapsed favorite color categories.

```
chisq.test(table(survey1$sex, survey1$color.new2))
```

Pearson's Chi-squared test

```
data: table(survey1$sex, survey1$color.new2)
X-squared = 5, df = 3, p-value = 0.2
```

## 32.6 Accuracy of Death Certificates (A 6x3 Table)

The table below compiles data from six studies designed to investigate the accuracy of death certificates<sup>1</sup>. 5373 autopsies were compared to the causes of death listed on the certificates. Of those, 3726 were confirmed to be accurate, 783 either lacked information or contained inaccuracies but did not require recoding of the underlying cause of death, and 864 were incorrect and required recoding. Do the results across studies appear consistent?

Date of Study	[Confirmed]	Accurate	[Inaccurate]	No Change	[Incorrect]	Recoding	Total
1955-1965		2040		367		327	2734

<sup>1</sup>Source: Pagano M, Gauvreau K (2000) Principles of Biostatistics, 2nd Edition, Pacific Grove, CA: Duxbury, pp. 367-8. The original citation is Kircher T, Nelson J, Burdo H (1985) The autopsy as a measure of accuracy of the death certificate. NEJM, 313, 1263-1269.

Date of Study	[Confirmed]	Accurate	[Inaccurate]	No Change	[Incorrect]	Recoding	Total
1970	149		60		48		257
1970-1971	288		25		70		383
1975-1977	703		197		252		1152
1977-1978	425		62		88		575
1980	121		72		79		272
Total	3726		783		864		5373

## 32.7 The Pearson Chi-Square Test of Independence

We can assess the homogeneity of the confirmation results (columns) we observe in the table using a Pearson chi-squared test of independence.

- The null hypothesis is that the rows and columns are independent.
- The alternative hypothesis is that there is an association between the rows and the columns.

```
z <- matrix(c(2040, 367, 327, 149, 60, 48, 288, 25, 70, 703,
             197, 252, 425, 62, 88, 121, 72, 79), byrow=TRUE, nrow=6)
rownames(z) <- c("1955-65", "1970", "1970-71", "1975-77", "1977-78",
                  "1980")
colnames(z) <- c("Confirmed", "Inaccurate", "Incorrect")

addmargins(z)
```

	Confirmed	Inaccurate	Incorrect	Sum
1955-65	2040	367	327	2734
1970	149	60	48	257
1970-71	288	25	70	383
1975-77	703	197	252	1152
1977-78	425	62	88	575
1980	121	72	79	272
Sum	3726	783	864	5373

To see the potential heterogeneity across rows in these data, we should perhaps also look at the proportions of autopsies in each of the three accuracy categories for each study.

```
addmargins(round(100*prop.table(z, 1), 1), 2)
```

	Confirmed	Inaccurate	Incorrect	Sum
1955-65	74.6	13.4	12.0	100
1970	58.0	23.3	18.7	100
1970-71	75.2	6.5	18.3	100
1975-77	61.0	17.1	21.9	100
1977-78	73.9	10.8	15.3	100
1980	44.5	26.5	29.0	100

In three of the studies, approximately 3/4 of the results were confirmed. In the other three, 45%, 58% and 61% were confirmed. It looks like there's a fair amount of variation in results across studies. To see if this is true, formally, we run Pearson's chi-square test of independence, where the null hypothesis is that the rows and columns are independent, and the alternative hypothesis is that there is an association between the rows and the columns.

```
chisq.test(z)
```

**Pearson's Chi-squared test**

```
data: z
X-squared = 200, df = 10, p-value <2e-16
```

The chi-square test statistic is 200 on 10 degrees of freedom, yielding  $p < 0.0001$ .

Autopsies are not performed at random; in fact, many are done because the cause of death listed on the certificate is uncertain. What problems may arise if you attempt to use the results of these studies to make inference about the population as a whole?

# Chapter 33

## Three-Way Tables: A 2x2xK Table and a Mantel-Haenszel Analysis

The material I discuss in this section is attributable to Jeff Simonoff and his book *Analyzing Categorical Data*. The example is taken from Section 8.1 of that book.

A three-dimensional or three-way table of counts often reflects a situation where the rows and columns refer to variables whose association is of primary interest to us, and the third factor (a layer, or strata) describes a control variable, whose effect on our primary association is something we are *controlling* for in the analysis.

### 33.1 Smoking and Mortality in the UK

In the early 1970s and then again 20 years later, in Whickham, United Kingdom, surveys yielded the following relationship between whether a person was a smoker at the time of the original survey and whether they were still alive 20 years later<sup>1</sup>.

```
whickham1 <- matrix(c(443, 139, 502, 230), byrow=TRUE, nrow=2)
rownames(whickham1) <- c("Smoker", "Non-Smoker")
colnames(whickham1) <- c("Alive", "Dead")
pander(addmargins(whickham1))
```

	Alive	Dead	Sum
Smoker	443	139	582
Non-Smoker	502	230	732
Sum	945	369	1314

Here's the two-by-two table analysis.

```
twoby2(whickham1)
```

2 by 2 table analysis:

```
-----  
Outcome : Alive  
Comparing : Smoker vs. Non-Smoker
```

<sup>1</sup>See Appleton et al. 1996. Ignoring a Covariate: An Example of Simpson's Paradox. *The American Statistician*, 50, 340-341.

	Alive	Dead	P(Alive)	95% conf. interval
Smoker	443	139	0.761	0.725    0.794
Non-Smoker	502	230	0.686	0.651    0.718
			95% conf. interval	
Relative Risk:	1.1099		1.0381    1.187	
Sample Odds Ratio:	1.4602		1.1414    1.868	
Conditional MLE Odds Ratio:	1.4598		1.1335    1.884	
Probability difference:	0.0754		0.0266    0.123	
			Exact P-value: 0.003	
			Asymptotic P-value: 0.0026	

---

```
chisq.test(whickham1)
```

Pearson's Chi-squared test with Yates' continuity correction

```
data: whickham1
X-squared = 9, df = 1, p-value = 0.003
```

There is a significant association between smoking and mortality ( $\chi^2 = 8.75$  on 1 df,  $p = 0.003$ ), but it isn't the one you might expect.

- The odds ratio is 1.46, implying that the odds of having lived were 46% higher for smokers than for non-smokers.
- Does that mean that smoking is *good* for you?

Not likely. There is a key “lurking” variable here - a variable that is related to both smoking and mortality that is obscuring the actual relationship - namely, age.

## 33.2 The `whickham` data including age, as well as smoking and mortality

The table below gives the mortality experience separated into subtables by initial age group.

```
age <- c(rep("18-24", 4), rep("25-34", 4),
      rep("35-44", 4), rep("45-54", 4),
      rep("55-64", 4), rep("65-74", 4),
      rep("75+", 4))
smoking <- c(rep(c("Smoker", "Smoker", "Non-Smoker", "Non-Smoker"), 7))
status <- c(rep(c("Alive", "Dead"), 14))
counts <- c(53, 2, 61, 1, 121, 3, 152, 5,
           95, 14, 114, 7, 103, 27, 66, 12,
           64, 51, 81, 40, 7, 29, 28, 101,
           0, 13, 0, 64)
whickham2 <- data.frame(smoking, status, age, counts) %>% tbl_df()
whickham2$smoking <- factor(whickham2$smoking, levels = c("Smoker", "Non-Smoker"))
whickham2.tab1 <- xtabs(counts ~ smoking + status + age, data = whickham2)
whickham2.tab1

, , age = 18-24

      status
```

smoking	Alive	Dead
Smoker	53	2
Non-Smoker	61	1

, , age = 25-34

smoking	Alive	Dead
Smoker	121	3
Non-Smoker	152	5

, , age = 35-44

smoking	Alive	Dead
Smoker	95	14
Non-Smoker	114	7

, , age = 45-54

smoking	Alive	Dead
Smoker	103	27
Non-Smoker	66	12

, , age = 55-64

smoking	Alive	Dead
Smoker	64	51
Non-Smoker	81	40

, , age = 65-74

smoking	Alive	Dead
Smoker	7	29
Non-Smoker	28	101

, , age = 75+

smoking	Alive	Dead
Smoker	0	13
Non-Smoker	0	64

The odds ratios for each of these subtables, except the last one, where it is undefined are as follows:

Age Group	Odds Ratio
18-24	0.43
25-34	1.33
35-44	0.42
45-54	0.69
55-64	0.62

Age Group	Odds Ratio
65-74	0.87
75+	undefined

Thus, for all age groups except 25-34 year olds, smoking is associated with higher mortality.

Why? Not surprisingly, there is a strong association between age and mortality, with mortality rates being very low for young people (2.5% for 18-24 year olds) and increasing to 100% for 75+ year olds.

There is also an association between age and smoking, with smoking rates peaking in the 45-54 year old range and then falling off rapidly. In particular, respondents who were 65 and older at the time of the first survey had very low smoking rates (25.4%) but very high mortality rates (85.5%). Smoking was hardly the cause, however, since even among the 65-74 year olds mortality was higher among smokers (80.6%) than it was among non-smokers (78.3%). A flat version of the table (`ftable` in R) can help us with these calculations.

```
ftable(whickham2.tab1)
```

		age	18-24	25-34	35-44	45-54	55-64	65-74	75+
smoking	status								
Smoker	Alive		53	121	95	103	64	7	0
	Dead		2	3	14	27	51	29	13
Non-Smoker	Alive		61	152	114	66	81	28	0
	Dead		1	5	7	12	40	101	64

### 33.2.1 The Cochran-Mantel-Haenszel Test

So, the marginal table looking at smoking and mortality combining all age groups isn't the most meaningful summary of the relationship between smoking and mortality. Instead, we need to look at the *conditional* association of smoking and mortality, `given age`, to address our interests.

The null hypothesis would be that, in the population, smoking and mortality are independent within strata formed by age group. In other words,  $H_0$  requires that smoking be of no value in predicting mortality once age has been accounted for.

The alternative hypothesis would be that, in the population, smoking and mortality are associated within the strata formed by age group. In other words,  $H_A$  requires that smoking be of at least some value in predicting mortality even after age has been accounted for.

We can consider the evidence that helps us choose between these two hypotheses with a Cochran-Mantel-Haenszel test, which is obtained in R through the `mantelhaen.test` function. This test requires us to assume that, in the population and within each age group, the smoking-mortality odds ratio is the same. Essentially, this means that the association of smoking with mortality is the same for older and younger people.

```
mantelhaen.test(whickham2.tab1, conf.level = 0.90)
```

```
Mantel-Haenszel chi-squared test with continuity correction
```

```
data: whickham2.tab1
Mantel-Haenszel X-squared = 5, df = 1, p-value = 0.02
alternative hypothesis: true common odds ratio is not equal to 1
90 percent confidence interval:
 0.490 0.875
sample estimates:
common odds ratio
 0.655
```

- The Cochran-Mantel-Haenszel test statistic is 5.44 (after a continuity correction) leading to a  $p$  value of 0.02, indicating strong rejection of the null hypothesis of conditional independence of smoking and survival given age.
- The estimated common conditional odds ratio is 0.65. This implies that (given age) being a smoker is associated with a 35% lower odds of being alive 20 years later than being a non-smoker.
- A 90% confidence interval for that common odds ratio is (0.49, 0.87), reinforcing rejection of the conditional independence (where the odds ratio would be 1).

### 33.2.2 Checking Assumptions: The Woolf test

We can also obtain a test (using the `woolf_test` function, in the `vcd` library) to see if the common odds ratio estimated in the Mantel-Haenszel procedure is reasonable for all age groups. In other words, the Woolf test is a test of the assumption of homogeneous odds ratios across the six age groups.

If the Woolf test is significant, it suggests that the Cochran-Mantel-Haenszel test is not appropriate, since the odds ratios for smoking and mortality vary too much in the sub-tables by age group. Here, we have the following log odds ratios (estimated using conditional maximum likelihood, rather than cross-product ratios) and the associated Woolf test.

```
## Next two results use the vcd library

vcd::oddsratio(whickham2.tab1, log = TRUE)

log odds ratios for smoking and status by age

 18-24   25-34   35-44   45-54   55-64   65-74     75+
-0.6502  0.2247 -0.8407 -0.3461 -0.4742 -0.0993  1.5640
```

```
Woolf-test on Homogeneity of Odds Ratios (no 3-Way assoc.)
```

```
data: whickham2.tab1
X-squared = 3, df = 6, p-value = 0.8
```

As you can see, the Woolf test is not close to statistically significant, implying the common odds ratio is at least potentially reasonable for all age groups (or at least the ones under ages 75, where some data are available.)

### 33.2.3 Without the Continuity Correction

By default, R presents the Mantel-Haenszel test with a continuity correction, when used for a 2x2xK table. In virtually all cases, go ahead and do this, but as you can see below, the difference it makes in this case is modest.

```
mantelhaen.test(whickham2.tab1, correct=FALSE, conf.level = 0.90)
```

```
Mantel-Haenszel chi-squared test without continuity correction
```

```
data: whickham2.tab1
Mantel-Haenszel X-squared = 6, df = 1, p-value = 0.02
alternative hypothesis: true common odds ratio is not equal to 1
90 percent confidence interval:
 0.490 0.875
```

```
sample estimates:  
common odds ratio  
0.655
```

# Chapter 34

## Some Thoughts on *p* values

### 34.1 What does Dr. Love dislike about *p* values?

A lot of things. A major issue is that I believe that *p* values are impossible to explain in a way that is both [a] technically correct and [b] straightforward at the same time. As evidence of this, you might want to look at this article and associated video by Christie Aschwanden at 538.com

The notion of a *p* value was an incredibly impressive achievement back when Wald and others were doing the work they were doing in the 1940s, and might still have been useful as recently as 10 years ago. But the notion of a *p* value relies on a lot of flawed assumptions, and null hypothesis significance testing is fraught with difficulties. Nonetheless, researchers use *p* values every day.

For the moment, I will say this. I emphasize confidence intervals over *p* values, which is at best a partial solution. But ...

1. Very rarely does a situation emerge in which a *p* value can be available in which looking at the associated confidence interval isn't far more helpful for making a comparison of interest.
2. The use of a *p* value requires making at least as many assumptions about the population, sample, individuals and data as does a confidence interval.
3. Most null hypotheses are clearly not exactly true prior to data collection, and so the test summarized by a *p* value is of questionable value most of the time.
4. No one has a truly adequate definition of a *p* value, in terms of both precision and parsimony. Brief, understandable definitions always fail to be technically accurate.
5. Bayesian approaches avoid some of these pitfalls, but come with their own issues.
6. Many smart people agree with me, and use *p* values sparingly.

The American Statistical Association released a statement on the use and abuse of *p* values in March 2016, and we'll be discussing that statement and some of the reactions to it.

### 34.2 On Reporting *p* Values

When reporting a *p* value and no rounding rules are in place from the lead author/journal/source for publication, follow these conventions...

1. Use an italicized, lower-case *p* to specify the *p* value. Don't use *p* for anything else.
2. For *p* values above 0.10, round to two decimal places, at most.
3. For *p* values near  $\alpha$ , include only enough decimal places to clarify the reject/retain decision.
4. For very small *p* values, always report either  $p < 0.0001$  or even just  $p < 0.001$ , rather than specifying the result in scientific notation, or, worse, as  $p = 0$  which is glaringly inappropriate.

5. Report  $p$  values above 0.99 as  $p > 0.99$ , rather than  $p = 1$ .

### **34.3 Much more to come, in class.**

## **Chapter 35**

# **Study Design: Type S and Type M Errors**

### **35.1 Materials to come.**

Dr. Love will add materials here, soon.



# Chapter 36

## Partial Review to help you prepare for Quiz 2

There are 15 items here, and then a set of answer sketches follow the questions. This **isn't a complete review** - there are no questions here about either ANOVA or Mantel-Haenszel methods, for instance, and each might show up on Quiz 2.

### 36.1 Review Items 1-7

Researchers comparing the effectiveness of two pain medications randomly selected a group of patients who had been complaining of a certain kind of joint pain. They randomly divided those people into two groups, then administered the medications. Of the 85 people in the group who received medication A, 65 said that it provided relief. Of the 70 people in the group receiving medication B, 45 reported that it provided relief.

1. Use the single augmentation with an imaginary failure or success (SAIFS) approach to specify a 95% confidence interval for the proportion of people who find relief from this kind of joint pain by using medication A.
2. Now use the same approach to specify a 95% confidence interval for the proportion of people who find relief using medication B.
3. Do the confidence intervals in items 1 and 2 overlap? What conclusions can you draw in light of that overlap (or lack thereof) about whether medication A or medication B is significantly more effective?
4. Specify and display the correct 2x2 table (incorporating a Bayesian augmentation) analysis to enable you to study the A - B difference in the true proportions of people who find these medications effective.
5. Use the 2x2 table results to specify an appropriate odds ratio and its 95% confidence interval in this situation, and explain what the values mean in context.
6. Specify the hypotheses ( $H_0$  and  $H_A$ ) tested by the Fisher exact test you obtain in your 2x2 table. What does the provided  $p$  value tell you about what conclusion you should draw in this case regarding those hypotheses?
7. If you have made an error in your conclusion for item 6, was it a Type I error or a Type II error? How do you know?

### 36.2 Review Items 8-9

For each of the following statements, indicate whether or not the statement is true or false, and specify how you know.

8. If there is sufficient evidence to reject a null hypothesis at the 10% level, then there is sufficient evidence to reject it at the 5% level.
9. A sample histogram will follow a normal distribution if the sample size is large enough.

### 36.3 Review Items 10-13

Charles Darwin carried out an experiment to study whether seedlings from cross-fertilized plants tend to be superior to those from self-fertilized plants. He covered a number of plants with fine netting so that insects would be unable to fertilize them. He fertilized a number of flowers on each plant with their own pollen and he fertilized an equal number of flowers on the same plant with pollen from a distant plant. (He did not specify how he decided which flowers received which treatments.) The seeds from the flowers were allowed to ripen and were set in wet sand to germinate. He placed two seedlings of the same age in a pot, one from a seed from a self-fertilized flower and one from a cross-fertilized flower.

He repeated this process with a total of 15 such pots. Each pot was then set aside for a time, so that the two plants in the plot would receive similar exposure to atmospheric conditions (sun, rainfall, etc.). Later, he gathered the heights of the plants (in inches) that came from those 15 cross-fertilized and 15 self-fertilized seeds at certain points in time. Those data are contained in the `darwin.csv` data set on our course website.

10. Does this study call for a paired samples or independent samples comparison? How do you know?
11. Display and interpret an appropriate graph to determine whether a t-test or a Wilcoxon test would be more appropriate for these data.
12. Use the method (t or Wilcoxon) you specified in item 11 to find an appropriate 95% confidence interval for the average height difference between cross-fertilized and self-fertilized seedlings. Verify that your confidence interval describes the “cross” - “self” difference, rather than the opposite direction.
13. Use an appropriate bootstrap procedure (setting your random seed to be 4310) to provide an alternative answer for the question posed in item 12. Is this bootstrap confidence interval wider or narrower than the interval you produced in item 12?

### 36.4 Review Items 14-15

You have been asked how large a sample size will be required for a clinical trial comparing two different approaches to blood pressure control. In approach A, we believe that the average systolic blood pressure will drop by 7 mm Hg, on the basis of our prior work in this area, while in the new approach B, we hope to see a clinically meaningful additional decline - specifically, we are looking for at least a 50% larger decline, so that the average systolic blood pressure will drop by 10.5 mm Hg or more over the same amount of time. Thus, the minimum clinically meaningful difference we are looking for is 3.5 mm Hg. Suppose we believe that the relevant standard deviation is 9 mm Hg, and we want to complete the trial using a 5% significance level and a two-sided t test.

14. What will be the power of the test if we have a balanced design with 120 subjects in approach A and 120 different subjects in approach B? Show your calculation, and state your final result in a sentence.
15. What is the smallest total sample size that we can use in a balanced design to maintain at least 90% power to detect the difference of interest, while still using independent samples? Show your calculation, and state your final result in a sentence.

## 36.5 Answer Sketch for Review Items

### 36.5.1 Answer 1

```
saifs.ci(65, 85)
```

Sample Proportion	0.025	0.975
	0.765	0.859

The 95% confidence interval for the proportion of people using medication A who obtain relief is (0.663, 0.859). We are 95% confident that the true percentage of people who find relief using medication A is between 66.3% and 85.9%.

### 36.5.2 Answer 2

```
saifs.ci(45, 70)
```

Sample Proportion	0.025	0.975
	0.643	0.519

The 95% confidence interval for the proportion of people using medication B who obtain relief is (0.519, 0.762).

### 36.5.3 Answer 3

The confidence intervals do overlap, so we cannot conclude from the separate intervals that there is (or isn't) a statistically significant difference in the effectiveness rates for medications A and B. If the confidence intervals didn't overlap, then we would know that there was a statistically significant difference in effectiveness between the two medications.

### 36.5.4 Answer 4

```
twobytwo(65+1, 20+1, 45+1, 25+1, "Med. A", "Med. B", "Relief", "No Relief")
```

2 by 2 table analysis:

---

Outcome : Relief  
Comparing : Med. A vs. Med. B

	Relief	No Relief	P(Relief)	95% conf. interval
Med. A	66	21	0.759	0.658 0.837
Med. B	46	26	0.639	0.522 0.741

	95% conf. interval		
Relative Risk:	1.19	0.9623	1.465
Sample Odds Ratio:	1.78	0.8934	3.532
Conditional MLE Odds Ratio:	1.77	0.8446	3.749
Probability difference:	0.12	-0.0223	0.259

Exact P-value: 0.117  
Asymptotic P-value: 0.101

---

### 36.5.5 Answer 5

The odds ratio is 1.78, with 95% confidence interval (0.89, 3.53). The point estimate states that the odds of finding relief with medication A are 78% higher than the odds of finding relief with medication B. But the confidence interval indicates that, with 95% confidence, we can conclude only that the odds of relief with medication A are between 0.89 and 3.53 times as high as the odds of relief with medication B. Since 1 is in that confidence interval, we must conclude that there is no statistically significant effect of the medication choice on the odds of this outcome.

### 36.5.6 Answer 6

- $H_0$ : Medication Choice (A or B) is unrelated to the probability of Relief
- $H_A$ : Medication Choice and Relief are associated

The  $p$  value is 0.12, from the Fisher exact test. This means that we must retain the null hypothesis, and conclude that there is no significant association between Medication choice and the probability of Relief.

### 36.5.7 Answer 7

You would have made a Type II error. A Type II error can be made if you incorrectly retain  $H_0$ . Since we retain  $H_0$ , if we've made an error, it must have been a Type II error, since a Type I error occurs when you incorrectly reject  $H_0$ .

### 36.5.8 Answer 8

This is FALSE. Sufficient evidence to reject  $H_0$  at the 10% level means that we have a  $p$  value  $< 0.10$ . In order to have sufficient evidence to reject  $H_0$  at the 5% level, we'd need to have a  $p$  value  $< 0.05$ . If our  $p < 0.10$ , this doesn't guarantee that it is also true that  $p < 0.05$ .

### 36.5.9 Answer 9

Also FALSE. The mean of a sample will approach a Normal distribution, but if the data are skewed, the data will still be skewed no matter how many observations we see.

### 36.5.10 Answer 10

These samples are paired by the pot. Each pot provides a cross-fertilized seedling height and a self-fertilized seedling height. We should be comparing paired differences.

### 36.5.11 Answer 11

We need a plot of the 15 paired differences, for example a boxplot, or a normal Q-Q plot.

```
darwin$diffs <- darwin$cross.fertilized - darwin$self.fertilized

p1 <- ggplot(darwin, aes(x = diffs)) +
  geom_histogram(aes(y = ..density..), bins = fd_bins(darwin$diffs),
```

```

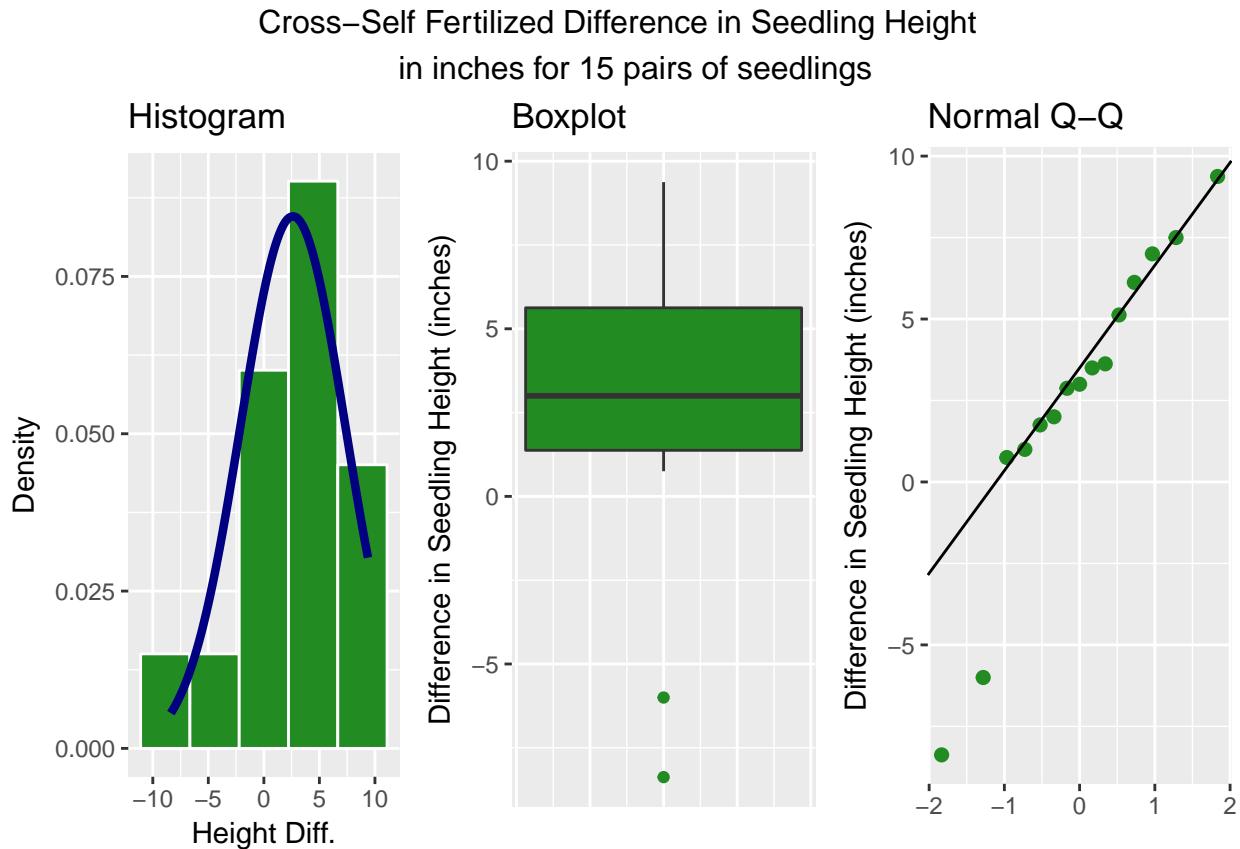
        fill = "forestgreen", col = "white") +
stat_function(fun = dnorm,
              args = list(mean = mean(darwin$diffs),
                          sd = sd(darwin$diffs)),
              lwd = 1.5, col = "navy") +
labs(title = "Histogram",
     x = "Height Diff.", y = "Density")

p2 <- ggplot(darwin, aes(x = 1, y = diffss)) +
geom_boxplot(fill = "forestgreen", outlier.color = "forestgreen") +
theme(axis.text.x = element_blank(), axis.ticks.x = element_blank()) +
labs(title = "Boxplot",
     y = "Difference in Seedling Height (inches)", x = "")

p3 <- ggplot(darwin, aes(sample = diffss)) +
geom_qq(col = "forestgreen", size = 2) +
geom_abline(intercept = qq_int(darwin$diffss),
            slope = qq_slope(darwin$diffss)) +
labs(title = "Normal Q-Q",
     y = "Difference in Seedling Height (inches)", x = "")

gridExtra::grid.arrange(p1, p2, p3, nrow=1,
                       top = "Cross-Self Fertilized Difference in Seedling Height
in inches for 15 pairs of seedlings")

```



It appears that we have two low outliers out of the 15 paired differences. Assuming normality seems

inappropriate here. I would probably use a Wilcoxon approach instead.

### 36.5.12 Answer 12

```
wilcox.test(darwin$diffs, conf.int=TRUE, conf.level=0.95)
```

Wilcoxon signed rank test

```
data: darwin$diffs
V = 100, p-value = 0.04
alternative hypothesis: true location is not equal to 0
95 percent confidence interval:
0.50 5.19
sample estimates:
(pseudo)median
3.12
```

The cross-self differences appear to have a population pseudomedian which we are 95% confident is between 0.5 and 5.2 inches. The cross-fertilized plants appear to be statistically significantly taller on average than the self-fertilized plants in the same pot.

### 36.5.13 Answer 13

```
set.seed(4310); smean.cl.boot(darwin$diffs)
```

Mean	Lower	Upper
2.617	0.208	4.734

This confidence interval is a bit narrower than the interval in item 12, and also shifted a bit closer to zero. We are 95% confident that the population mean cross-self difference is between 0.2 and 4.7 inches.

### 36.5.14 Answer 14

```
power.t.test(n = 120, delta = 3.5, sd = 9, sig.level = 0.05)
```

Two-sample t test power calculation

```
n = 120
delta = 3.5
sd = 9
sig.level = 0.05
power = 0.851
alternative = two.sided
```

NOTE: n is number in \*each\* group

Such a test will have just over 85% power to detect the specified minimum clinically meaningful difference of 3.5 mm Hg, using a 5% two-sided significance level.

### 36.5.15 Answer 15

```
power.t.test(power=0.9, delta = 3.5, sd = 9, sig.level = 0.05)
```

Two-sample t test power calculation

```
n = 140
delta = 3.5
sd = 9
sig.level = 0.05
power = 0.9
alternative = two.sided
```

NOTE: n is number in \*each\* group

The minimum sample size we'll need is 140 subjects in each approach (A and B), so that's a total sample size of 280, to achieve 90% or higher power for the specified test while still using independent samples.



## **Part C. Building Models**



# Chapter 37

## Introduction for Part C

In 431, my primary goal is to immerse you in several cases, which will demonstrate good statistical practice in the analysis of data using multiple regression models. Often, we will leave gaps for 432, but the principal goal is to get you to the point where you can do a solid (if not quite complete) analysis of data for the modeling part of your project.

The ten main topics to be discussed or reviewed in these notes are:

1. Describing the multivariate relationship
  - a. Scatterplots and smoothing
  - b. Correlation coefficients, Correlation matrices
2. Transformations and Re-expression
  - a. The need for transformation
  - b. Using a Box-Cox method to help identify effective transformation choices
3. Testing the significance of a multiple regression model
  - a. T tests for individual predictors as last predictor in
  - b. Global F tests based on ANOVA to assess overall predictive significance
  - c. Incremental and Sequential testing of groups of predictors
4. Interpreting the predictive value of a model
  - a.  $R^2$  and Adjusted  $R^2$ , along with AIC and BIC
  - b. Residual standard deviation and RMSE
  - c. Estimating the effect size in terms of raw units, standard deviations or IQRs
  - d. Fitted values; Distinguishing prediction from confidence intervals
5. Checking model assumptions
  - a. Residual Analysis including studentized residuals, and the major plots
  - b. Identifying points with high Leverage
  - c. Assessing Influence numerically and graphically
  - d. Measuring and addressing collinearity
6. Model Selection
  - a. The importance of parsimony
  - b. Stepwise regression and other automated techniques
7. Assessing Predictive Accuracy through Cross-Validation
  - a. Summaries of predictive error
8. Dealing with Missing Values sensibly
  - a. Imputation vs. Complete Case analyses
  - b. Including a missing data category vs. simple imputation vs. removal
9. Dealing with Categorical Predictors
  - a. Indicator variables
  - b. Impact of Categorical Variables on the rest of our Modeling
10. Summarizing the Key Findings of the Model, briefly and accurately

- a. Making the distinction between causal findings and associations
- b. The importance of logic, theory and empirical evidence. (LTE)

## 37.1 Additional Reading

Vittinghoff et al. (2012) is strong in this area. The relevant sections of the text for 431 Part C are

- Section 3.3 on the Simple Linear Regression Model
- Chapter 4 on Linear Regression, where most of the material is relevant to 431, although we'll postpone the discussion of cubic splines, mostly, to 432.
- Chapters 10 (Model Selection) in particular the alternatives to  $R^2$  in 10.1.3.2 and some of the material on cross-validation, though we'll do much more in 432.
- A little of Chapter 11 (Missing Data), specifically, section 11.1.1 and a little of section 11.3, although we'll do more on this in 432 as well.

## 37.2 Scatterplots

We have often accompanied our scatterplots with regression lines estimated by the method of least squares, and by loess smooths which permit local polynomial functions to display curved relationships, and occasionally presented in the form of a scatterplot matrix to enable simultaneous comparisons of multiple two-way associations.

## 37.3 Correlation Coefficients

By far the most commonly used is the Pearson correlation, which is a unitless (scale-free) measure of bivariate linear association for the variables X and Y, symbolized by  $r$ , and ranging from -1 to +1. The Pearson correlation is a function of the slope of the least squares regression line, divided by the product of the standard deviations of X and Y.

We have also mentioned the Spearman rank correlation coefficient, which is obtained by using the usual formula for a Pearson correlation, but on the ranks (1 = minimum,  $n$  = maximum, with average ranks are applied to the ties) of the X and Y values. This approach (running a correlation of the orderings of the data) substantially reduces the effect of outliers. The result still ranges from -1 to +1, with 0 indicating no linear association.

## 37.4 Fitting a Linear Model

We have fit several styles of linear model to date, including both *simple* regressions, where our outcome Y is modeled as a linear function of a single predictor X, and *multiple* regression models, where more than one predictor is used. Important elements of a regression fit, obtained through the `summary` function for a `lm` object, include

- the estimated coefficients (intercept and slope(s)) of the fitted model, and
- the  $R^2$  or coefficient of determination, which specifies the proportion of variation in our outcome accounted for by the linear model.

## 37.5 Building Predictions from a Linear Model

We've also used the `predict` function applied to a `lm` object to obtain point and interval estimates for our outcome based on new values of the predictor(s). We've established both *confidence intervals* from such models, which describe the mean result across a population of subjects with the new predictor values, and *prediction intervals* which describe an individual result for a new subject with those same new values. Prediction intervals are much wider than confidence intervals.

## 37.6 Data Sets for Part C

```
hydrate <- read.csv("data/hydrate.csv") %>%tbl_df
hersirace <- read.csv("data/hersirace.csv") %>%tbl_df
wcgs <- read.csv("data/wcgs.csv") %>%tbl_df
emp_bmi <- read.csv("data/emp_bmi.csv") %>%tbl_df
gala <- read.csv("data/gala.csv") %>%tbl_df
```



# Chapter 38

## Re-Expression, Tukey’s Ladder & Box-Cox Plot

### 38.1 “Linearize” The Association between Quantitative Variables

Confronted with a scatterplot describing a monotone association between two quantitative variables, we may decide the data are not well approximated by a straight line, and thus, that a least squares regression may not be sufficiently useful. In these circumstances, we have at least two options, which are not mutually exclusive:

- Let the data be as they may, and summarize the scatterplot using tools like loess curves, polynomial functions, or cubic splines to model the relationship.
- Consider re-expressing the data (often we start with re-expressions of the outcome data [the Y variable]) using a transformation so that the transformed data may be modeled effectively using a straight line.

### 38.2 A New Tool: the Box-Cox Plot

As before, Tukey’s ladder of power transformations can guide our exploration.

Power ( $\lambda$ )	-2	-1	-1/2	0	1/2	1	2
Transformation	$1/y^2$	$1/y$	$1/\sqrt{y}$	$\log y$	$\sqrt{y}$	$y$	$y^2$

The **Box-Cox plot**, from the `boxCox` function in the `car` package, sifts through the ladder of options to suggest a transformation (for Y) to best linearize the outcome-predictor(s) relationship.

#### 38.2.1 A Few Caveats

- These methods work well with *monotone* data, where a smooth function of Y is either strictly increasing, or strictly decreasing, as X increases.
- Some of these transformations require the data to be positive. We can rescale the Y data by adding a constant to every observation in a data set without changing shape.
- We can use a natural logarithm (`log` in R), a base 10 logarithm (`log10`) or even sometimes a base 2 logarithm (`log2`) to good effect in Tukey’s ladder. All affect the association’s shape in the same way, so we’ll stick with `log` (base e).

4. Some re-expressions don't lead to easily interpretable results. Not many things that make sense in their original units also make sense in inverse square roots. There are times when we won't care, but often, we will.
5. If our primary interest is in making predictions, we'll generally be more interested in getting good predictions back on the original scale, and we can back-transform the point and interval estimates to accomplish this.

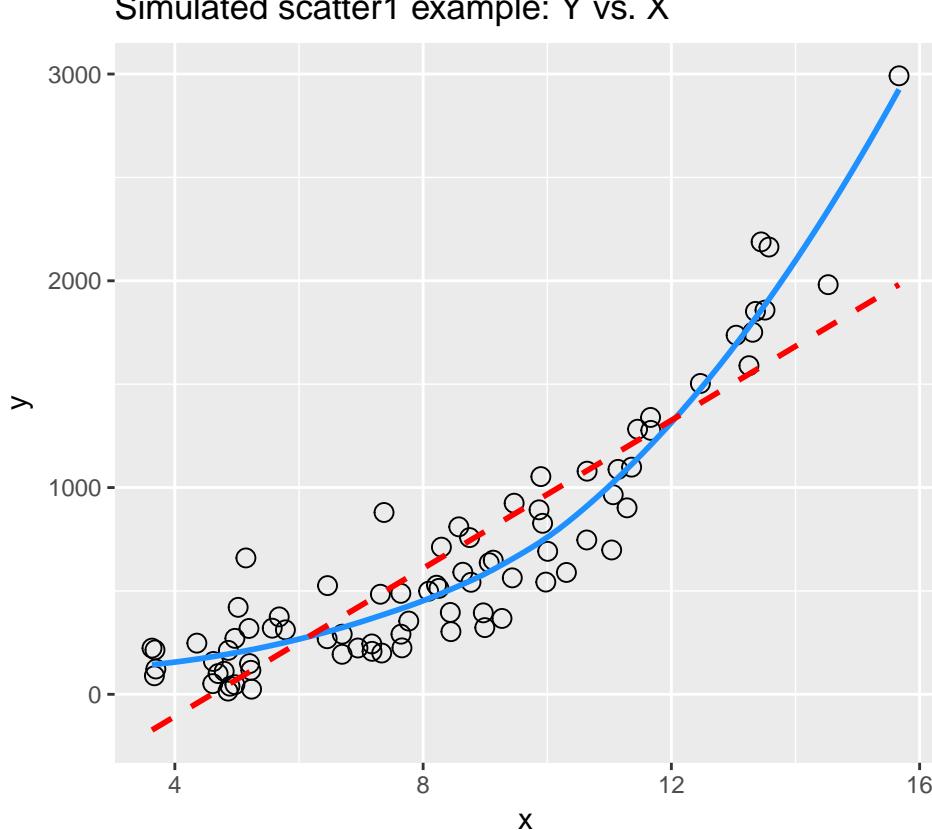
### 38.3 A Simulated Example

```

set.seed(999); x.rand <- rbeta(80, 2, 5) * 20 + 3
set.seed(1000); y.rand <- abs(50 + 0.75*x.rand^(3) - 0.65*x.rand + rnorm(80, 0, 200))
scatter1 <- data.frame(x = x.rand, y = y.rand) %>% tbl_df
rm(x.rand, y.rand)

ggplot(scatter1, aes(x = x, y = y)) +
  geom_point(shape = 1, size = 3) +
  ## add loess smooth
  geom_smooth(se = FALSE, col = "dodgerblue") +
  ## then add linear fit
  geom_smooth(method = "lm", se = FALSE, col = "red", linetype = "dashed") +
  labs(title = "Simulated scatter1 example: Y vs. X")
`geom_smooth()` using method = 'loess'

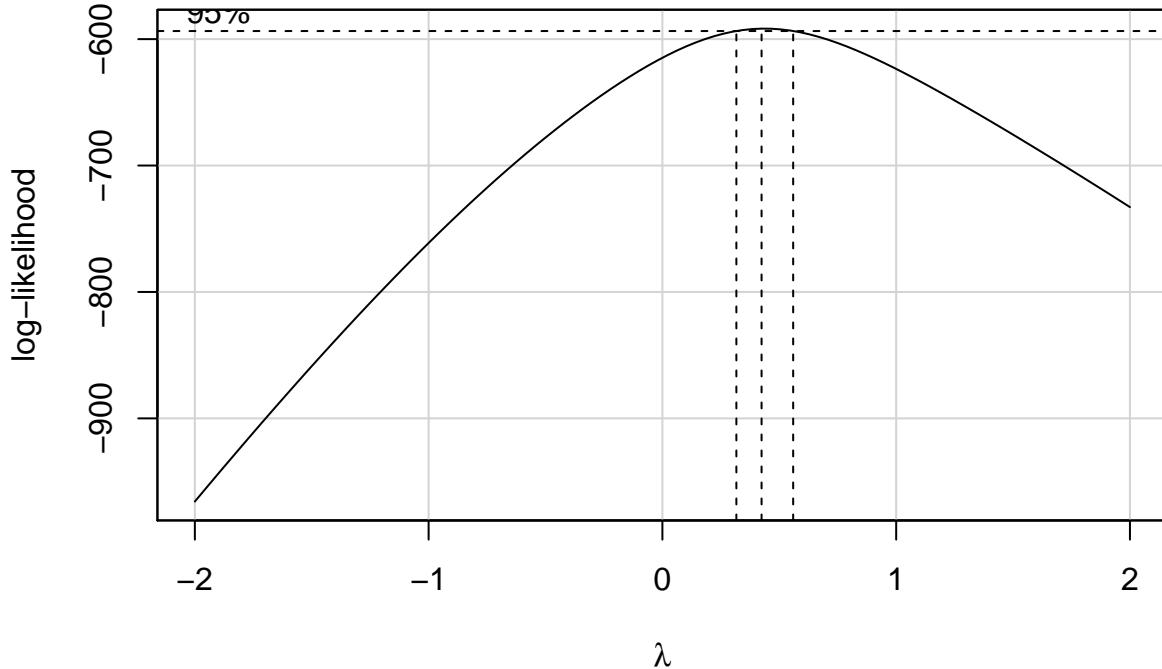
```



Having simulated data that produces a curved scatterplot, I will now use the Box-Cox plot to lead my choice

of an appropriate power transformation for Y in order to “linearize” the association of Y and X.

```
library(car)
boxCox(scatter1$y ~ scatter1$x)
```



```
powerTransform(scatter1$y ~ scatter1$x)
```

Estimated transformation parameters

```
Y1  
0.437
```

The Box-Cox plot peaks at the value  $\lambda = 0.44$ , which is pretty close to  $\lambda = 0.5$ . Now, 0.44 isn't on Tukey's ladder, but 0.5 is.

Power ( $\lambda$ )	-2	-1	-1/2	0	1/2	1	2
Transformation	$1/y^2$	$1/y$	$1/\sqrt{y}$	$\log y$	$\sqrt{y}$	$y$	$y^2$

If we use  $\lambda = 0.5$ , on Tukey's ladder of power transformations, it suggests we look at the relationship between the square root of Y and X, as shown next.

```
p1 <- ggplot(scatter1, aes(x = x, y = y)) +
  geom_point(size = 2) +
  geom_smooth(se = FALSE, col = "dodgerblue") +
  geom_smooth(method = "lm", se = FALSE, col = "red", linetype = "dashed") +
  labs(title = "scatter1: Y vs. X")

p2 <- ggplot(scatter1, aes(x = x, y = sqrt(y))) +
```

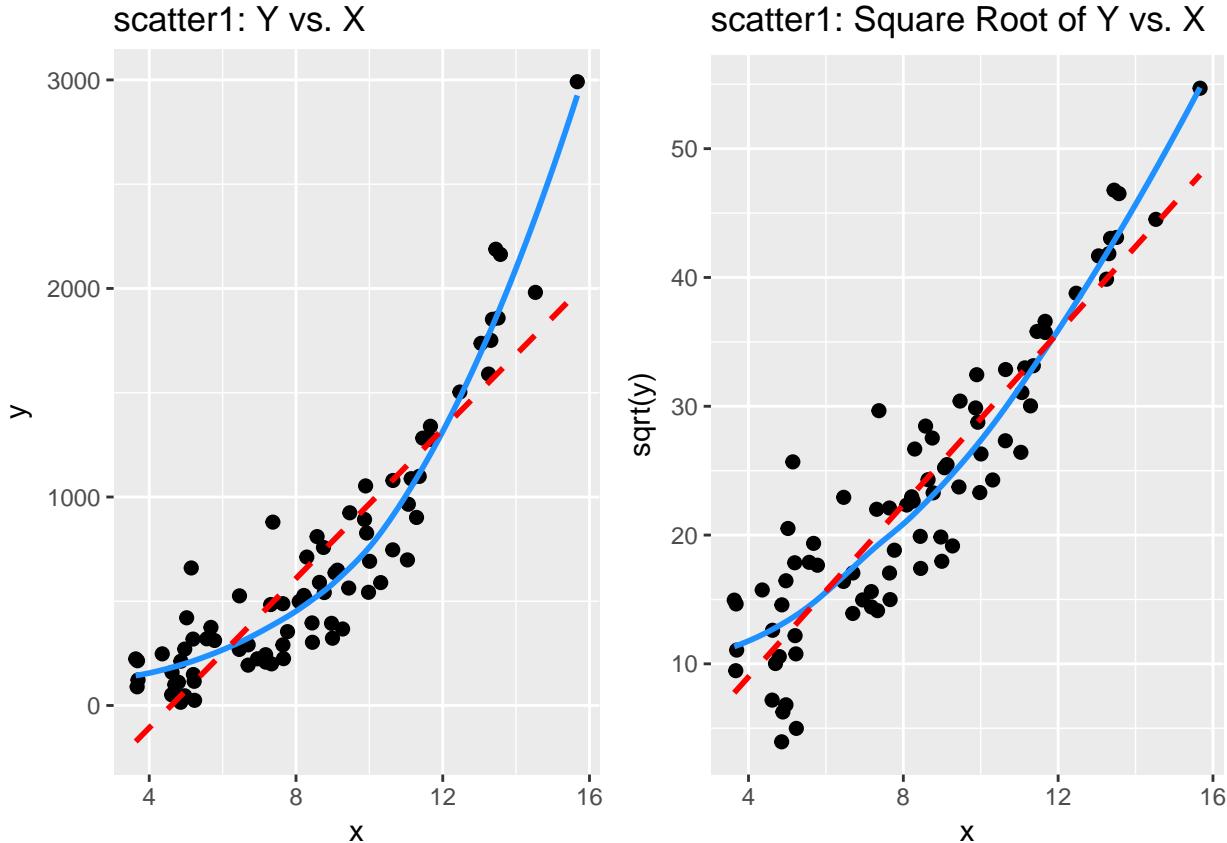
```

geom_point(size = 2) +
geom_smooth(se = FALSE, col = "dodgerblue") +
geom_smooth(method = "lm", se = FALSE, col = "red", linetype = "dashed") +
labs(title = "scatter1: Square Root of Y vs. X")

gridExtra::grid.arrange(p1, p2, nrow = 1)

`geom_smooth()` using method = 'loess'
`geom_smooth()` using method = 'loess'

```



By eye, I think the square root plot better matches the linear fit.

## 38.4 Checking on a Transformation or Re-Expression

We can do three more things to check on our transformation.

1. We can calculate the correlation of our original and re-expressed associations.
2. We can use the `testTransform` function in the `car` library in R to perform a statistical test comparing the optimal choice of power ( $\lambda = 0.44$ ) to various other transformations.
3. We can go ahead and fit the regression models using each approach and compare the plots of studentized residuals against fitted values from the data to see if the re-expression reduces the curve in that residual plot, as well.

Option 3 is by far the most important in practice, and it's the one we'll focus on going forward, but we'll demonstrate all three here.

### 38.4.1 Checking the Correlation Coefficients

Here, we calculate the correlation of original and re-expressed associations.

```
cor(scatter1$y, scatter1$x)

[1] 0.891

cor(sqrt(scatter1$y), scatter1$x)

[1] 0.914
```

The Pearson correlation is a little stronger after the transformation. as we'd expect.

### 38.4.2 Using the `testTransform` function

Here, we use the `testTransform` function (also from the `car` package) to compare the optimal choice determined by the `powerTransform` function (here  $\lambda = 0.44$ ) to  $\lambda = 0$  (logarithm), 0.5 (square root) and 1 (no transformation).

```
testTransform(powerTransform(scatter1$y ~ scatter1$x), 0)

LRT df      pval
LR test, lambda = (0) 46.2  1 1.08e-11

testTransform(powerTransform(scatter1$y ~ scatter1$x), 0.5)

LRT df      pval
LR test, lambda = (0.5) 1.02  1 0.311

testTransform(powerTransform(scatter1$y ~ scatter1$x), 1)

LRT df      pval
LR test, lambda = (1) 63.8  1 1.44e-15
```

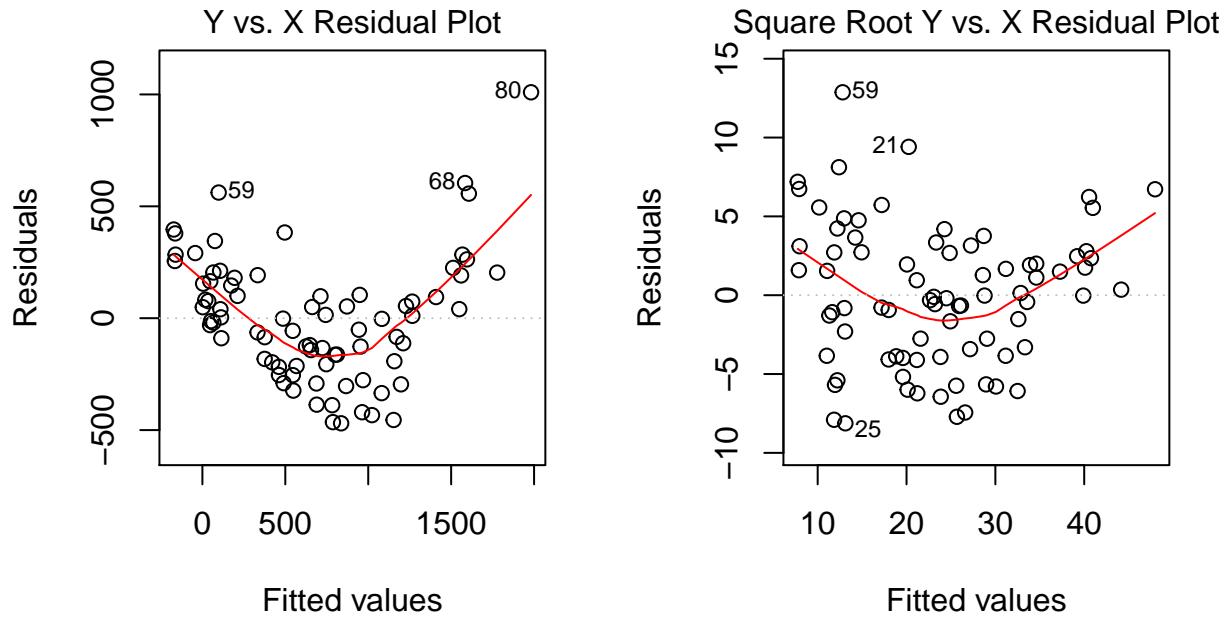
- It looks like only the square root ( $\lambda = 0.5$ ) of these three options is not significantly worse by the log-likelihood criterion applied here than the optimal choice.
- That's because it's the only one with a  $p$  value larger than our usual standard for statistical significance, of 0.05.

### 38.4.3 Comparing the Residual Plots

We can fit the regression models, obtain plots of residuals against fitted values, and compare them to see which one has less indication of a curve in the residuals.

```
model.orig <- lm(scatter1$y ~ scatter1$x)
model.sqrt <- lm(sqrt(scatter1$y) ~ scatter1$x)

par(mfrow=c(1,2))
plot(model.orig, which = 1, caption = "Y vs. X Residual Plot")
plot(model.sqrt, which = 1, caption = "Square Root Y vs. X Residual Plot")
```



```
par(mfrow=c(1,1))
```

What we're looking for in such a plot is the absence of a curve, among other things, we want to see "fuzzy football" shapes. The square root version, on the right, is a modest improvement in this regard over the original plot. It does look a bit less curved, and a bit more like a random cluster of points, so that's nice.

## Chapter 39

# Dehydration Recovery in Kids: A Small Study

The `hydrate` data describe the degree of recovery that takes place 90 minutes following treatment of moderate to severe dehydration, for 36 children diagnosed at a hospital's main pediatric clinic.

Upon diagnosis and study entry, patients were treated with an electrolytic solution at one of seven `dose` levels (0, 0.5, 1.0, 1.5, 2.0, 2.5, 3.0 mEq/l) in a frozen, flavored, ice popsicle. The degree of rehydration was determined using a subjective scale based on physical examination and parental input, converted to a 0 to 100 point scale, representing the percent of recovery (`recov.score`). Each child's `age` (in years) and `weight` (in pounds) are also available.

First, we'll check ranges (and for missing data) in the `hydrate` file.

```
hydrate
```

```
# A tibble: 36 x 5
  id recov.score dose   age weight
  <int>      <int> <dbl> <int> <int>
1 1          77    0.0    4     28
2 2          65    1.5    5     35
3 3          75    2.5    8     55
4 4          63    1.0    9     76
5 5          75    0.5    5     31
6 6          82    2.0    5     27
7 7          70    1.0    6     35
8 8          90    2.5    6     47
9 9          49    0.0    9     59
10 10        72    3.0    8     50
# ... with 26 more rows
```

```
summary(hydrate)
```

	id	recov.score	dose	age
Min. :	1.0	Min. : 44.0	Min. :0.00	Min. : 3.00
1st Qu.:	9.8	1st Qu.: 61.5	1st Qu.:1.00	1st Qu.: 5.00
Median :	18.5	Median : 71.5	Median :1.50	Median : 6.50
Mean   :	18.5	Mean   : 71.6	Mean   :1.57	Mean   : 6.67
3rd Qu.:	27.2	3rd Qu.: 80.0	3rd Qu.:2.50	3rd Qu.: 8.00
Max. :	36.0	Max. :100.0	Max. :3.00	Max. :11.00

weight

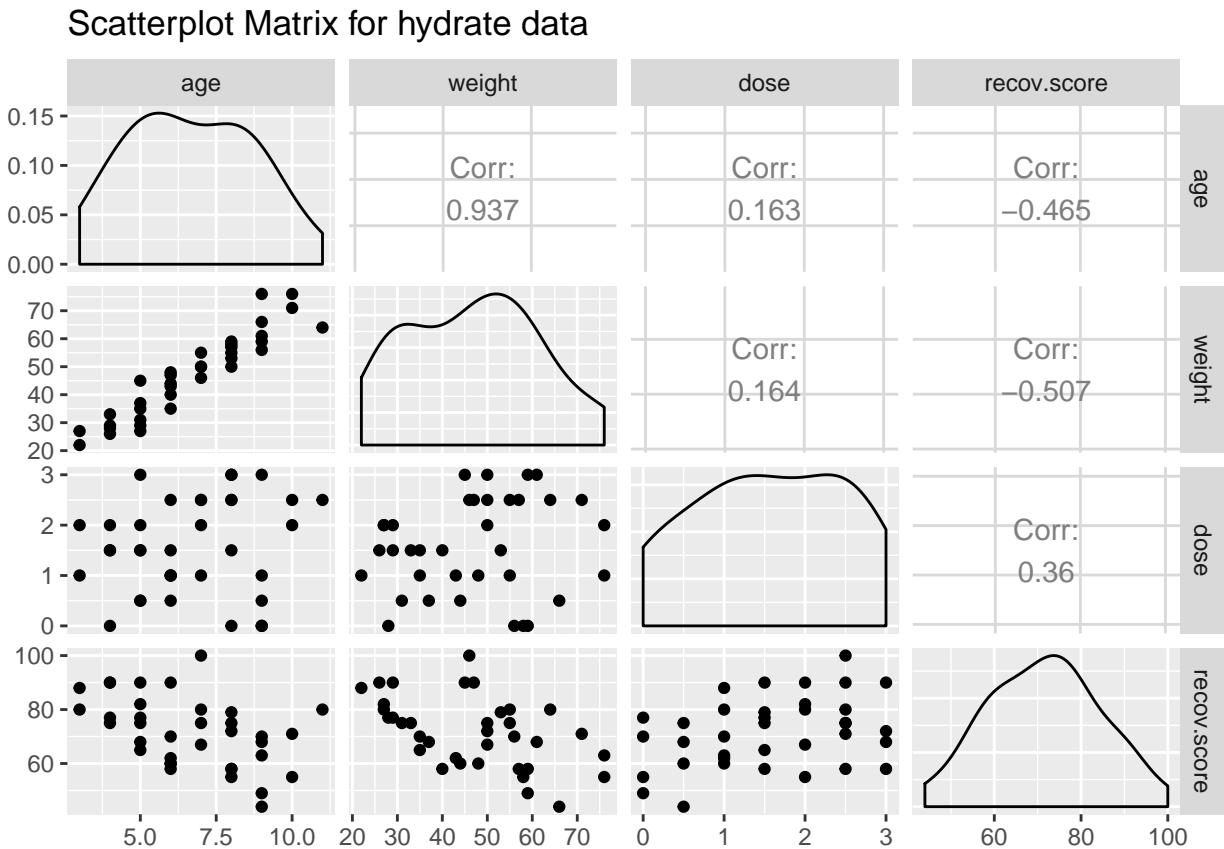
```
Min.    :22.0
1st Qu.:34.5
Median  :47.5
Mean    :46.9
3rd Qu.:57.2
Max.    :76.0
```

There are no missing values, and all of the ranges make sense. There are no especially egregious problems to report.

### 39.1 A Scatterplot Matrix

Next, we'll use a scatterplot matrix to summarize relationships between the outcome `recov.score` and the key predictor `dose` as well as the ancillary predictors `age` and `weight`, which are of less interest, but are expected to be related to our outcome. The one below uses the `ggpairs` function in the `GGally` package, as introduced in Part A of the Notes. We place the outcome in the bottom row, and the key predictor immediately above it, with `age` and `weight` in the top rows, using the `select` function within the '`ggpairs`' call.

```
GGally::ggpairs(dplyr::select(hydrate, age, weight, dose, recov.score),
                 title = "Scatterplot Matrix for hydrate data")
```



What can we conclude here?

- It looks like `recov.score` has a moderately strong negative relationship with both `age` and `weight` (with correlations in each case around -0.5), but a positive relationship with `dose` (correlation = 0.36).

- The distribution of `recov.score` looks to be pretty close to Normal. No potential predictors (`age`, `weight` and `dose`) show substantial non-Normality.
- `age` and `weight`, as we'd expect, show a very strong and positive linear relationship, with  $r = 0.94$
- Neither `age` nor `weight` shows a meaningful relationship with `dose`. ( $r = 0.16$ )

## 39.2 Are the recovery scores well described by a Normal model?

Next, we'll do a more thorough graphical summary of our outcome, recovery score, arranging the plots with the help of the `cowplot` package.

```
p1 <- ggplot(hydrate, aes(x = recov.score)) +
  geom_histogram(aes(y = ..density..),
                 bins = fd_bins(hydrate$recov.score),
                 fill = '#440154', col = '#FDE725') +
  stat_function(fun = dnorm,
                args = list(mean = mean(hydrate$recov.score),
                            sd = sd(hydrate$recov.score)),
                lwd = 1.5, col = '#1FA187') +
  labs(title = "Histogram", x = "Recovery Score", y = "") +
  theme_bw()

p2 <- ggplot(hydrate, aes(x = 1, y = recov.score)) +
  geom_boxplot(fill = '#FDE725', notch = TRUE,
               col = '#440154', outlier.color = '#440154') +
  labs(title = "Boxplot", x = "", y = "") +
  theme_bw() +
  theme(axis.text.x = element_blank(),
        axis.ticks.x = element_blank())

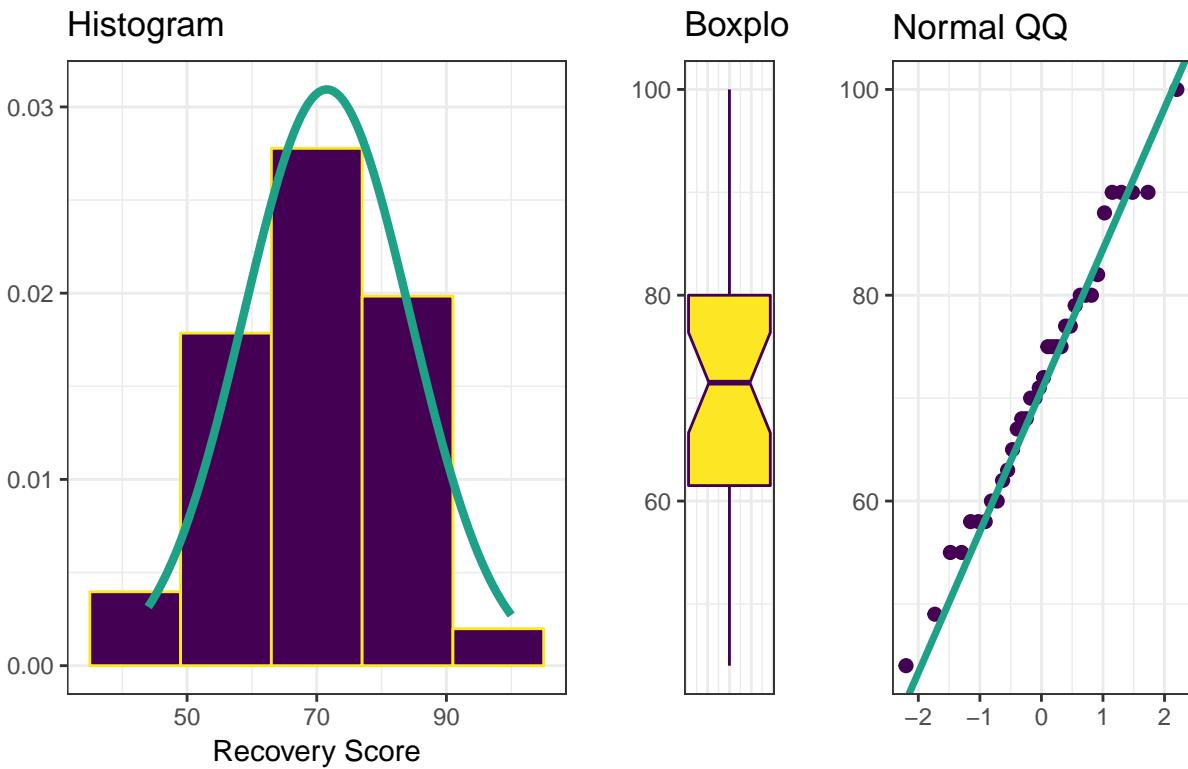
p3 <- ggplot(hydrate, aes(sample = recov.score)) +
  geom_qq(geom = "point", col = '#440154', size = 2) +
  geom_abline(slope = qq_slope(hydrate$recov.score),
              intercept = qq_int(hydrate$recov.score),
              col = '#1FA187', size = 1.25) +
  labs(title = "Normal QQ", x = "", y = "") +
  theme_bw()

p <- cowplot::plot_grid(p1, p2, p3, align = "h", nrow = 1,
                        rel_widths = c(3, 1, 2))

title <- cowplot::ggdraw() +
  cowplot::draw_label("Recovery Scores from 36 children in the hydrate study",
                      fontface = "bold")

cowplot::plot_grid(title, p, ncol = 1, rel_heights=c(0.1, 1))
```

### Recovery Scores from 36 children in the hydrate study



I see no serious problems with assuming Normality for these recovery scores. Our outcome variable doesn't in any way *need* to follow a Normal distribution, but it's nice when it does, because summaries involving means and standard deviations make sense.

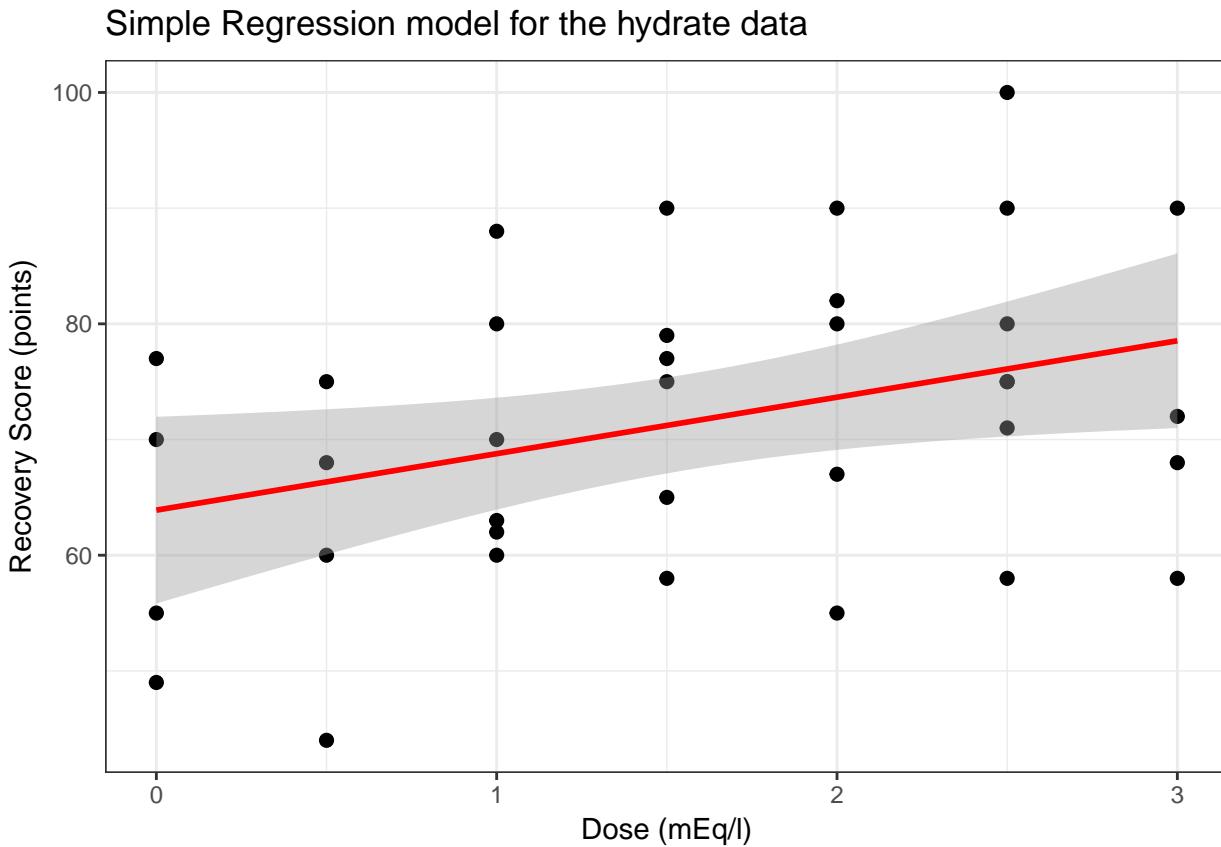
# Chapter 40

## Simple Regression: Using Dose to predict Recovery

To start, consider a simple (one predictor) regression model using `dose` alone to predict the % Recovery (`recov.score`). Ignoring the `age` and `weight` covariates, what can we conclude about this relationship?

### 40.1 The Scatterplot, with fitted Linear Model

```
ggplot(hydrate, aes(x = dose, y = recov.score)) +  
  geom_point(size = 2) +  
  geom_smooth(method = "lm", col = "red") +  
  theme_bw() +  
  labs(title = "Simple Regression model for the hydrate data",  
       x = "Dose (mEq/l)", y = "Recovery Score (points)")
```



## 40.2 The Fitted Linear Model

To obtain the fitted linear regression model, we use the `lm` function:

```
lm(recov.score ~ dose, data = hydrate)
```

```
Call:  
lm(formula = recov.score ~ dose, data = hydrate)
```

```
Coefficients:  
(Intercept)      dose  
       63.90        4.88
```

So, our fitted regression model (prediction model) is `recov.score = 63.9 + 4.88 dose`.

### 40.2.1 Confidence Intervals

We can obtain confidence intervals around the coefficients of our fitted model using the `confint` function.

```
confint(lm(recov.score ~ dose, data = hydrate))
```

	2.5 %	97.5 %
(Intercept)	55.827	72.0
dose	0.466	9.3

## 40.3 The Summary Output

To get a more complete understanding of the fitted model, we'll summarize it.

```
summary(lm(recov.score ~ dose, data = hydrate))
```

```
Call:
lm(formula = recov.score ~ dose, data = hydrate)
```

Residuals:

Min	1Q	Median	3Q	Max
-22.336	-7.276	0.063	8.423	23.903

Coefficients:

	Estimate	Std. Error	t value	Pr(> t )
(Intercept)	63.90	3.97	16.09	<2e-16 ***
dose	4.88	2.17	2.25	0.031 *
---				
Signif. codes:	0 '***'	0.001 '**'	0.01 '*'	0.05 '.'
	0.1	' '	' '	1

Residual standard error: 12.2 on 34 degrees of freedom

Multiple R-squared: 0.129, Adjusted R-squared: 0.104

F-statistic: 5.05 on 1 and 34 DF, p-value: 0.0313

### 40.3.1 Model Specification

1. The first part of the output specifies the model that has been fit.
  - Here, we have a simple regression model that predicts `recov.score` on the basis of `dose`.
  - Notice that we're treating `dose` here as a quantitative variable. If we wanted `dose` to be treated as a factor, we'd have specified that in the model.

### 40.3.2 Residual Summary

2. The second part of the output summarizes the regression **residuals** across the subjects involved in fitting the model.
  - The **residual** is defined as the Actual value of our outcome minus the predicted value of that outcome fitted by the model.
  - In our case, the residual for a given child is their actual `recov.score` minus the predicted `recov.score` according to our model, for that child.
  - The residual summary gives us a sense of how “incorrect” our predictions are for the `hydrate` observations.
    - A positive residual means that the observed value was higher than the predicted value from the linear regression model, so the prediction was too low.
    - A negative residual means that the observed value was lower than the predicted value from the linear regression model, so the prediction was too high.
    - The residuals will center near 0 (the ordinary least squares model fitting process is designed so the mean of the residuals will always be zero)
    - We hope to see the median of the residuals also be near zero, generally. In this case, the median prediction is 0.6 point too low.
    - The minimum and maximum show us the largest prediction errors, made in the subjects used to fit this model.

- Here, we predicted a recovery score that was 22.3 points too high for one patient, and another of our predicted recovery scores was 23.9 points too low.
- The middle half of our predictions were between 8.4 points too low and 7.3 points too high.

### 40.3.3 Coefficients Output

3. The **Coefficients** output begins with a table of the estimated coefficients from the regression equation.
  - Generally, we write a simple regression model as  $y = \beta_0 + \beta_1 x$ .
  - In the **hydrate** model, we have **recov.score** =  $\beta_0 + \beta_1$  **dose**.
  - The first column of the table gives the estimated  $\beta$  coefficients for our model
    - Here the estimated intercept  $\hat{\beta}_0 = 63.9$
    - The estimated slope of dose  $\hat{\beta}_1 = 4.88$
    - Thus, our model is **recov.score** =  $63.9 + 4.88$  **dose**

We interpret these coefficients as follows:

- The intercept (63.9) is the predicted **recov.score** for a patient receiving a **dose** of 0 mEq/l of the electrolytic solution.
- The slope (4.88) of the **dose** is the predicted *change* in **recov.score** associated with a 1 mEq/l increase in the dose of electrolytic solution.
  - Essentially, if we have two children like the ones studied here, and we give Roger a popsicle with dose X and Sarah a popsicle with dose X + 1, then this model predicts that Sarah will have a recovery score that is 4.88 points higher than will Roger.
  - From the confidence interval output we saw previously with the function **confint(lm(recov.score ~ dose))**, we are 95% confident that the true slope for **dose** is between (0.47, 9.30) mEq/l. We are also 95% confident that the true intercept is between (55.8, 72.0).

### 40.3.4 Correlation and Slope

If we like, we can use the **cor** function to specify the Pearson correlation of **recov.score** and **dose**, which turns out to be 0.36. - Note that the **slope** in a simple regression model will follow the sign of the Pearson correlation coefficient, in this case, both will be positive.

```
cor(hydrate$recov.score, hydrate$dose)
```

```
[1] 0.36
```

### 40.3.5 Coefficient Testing

```
summary(lm(recov.score ~ dose, data = hydrate))
```

```
Call:  
lm(formula = recov.score ~ dose, data = hydrate)
```

Residuals:

Min	1Q	Median	3Q	Max
-22.336	-7.276	0.063	8.423	23.903

Coefficients:

	Estimate	Std. Error	t value	Pr(> t )
(Intercept)	63.90	3.97	16.09	<2e-16 ***
dose	4.88	2.17	2.25	0.031 *

```
---
Signif. codes:  0 '***' 0.001 '**' 0.01 '*' 0.05 '.' 0.1 ' ' 1

Residual standard error: 12.2 on 34 degrees of freedom
Multiple R-squared:  0.129, Adjusted R-squared:  0.104
F-statistic: 5.05 on 1 and 34 DF,  p-value: 0.0313
```

Next to each coefficient in the summary regression table is its estimated standard error, followed by the coefficient's t value (the coefficient value divided by the standard error), and the associated two-tailed  $p$  value for the test of:

- $H_0$ : This coefficient's  $\beta$  value = 0 vs.
- $H_A$ : This coefficient's  $\beta$  value  $\neq 0$ .

For the slope coefficient, we can interpret this choice as:

- $H_0$ : This predictor adds no predictive value to the model vs.
- $H_A$ : This predictor adds statistically significant predictive value to the model.

The t test of the intercept is rarely of interest, because a. we rarely care about the situation that the intercept predicts, where all of the predictor variables are equal to zero and b. we usually are going to keep the intercept in the model regardless of its statistical significance.

In the `hydrate` simple regression model,

- the intercept is statistically significantly different from zero at all reasonable  $\alpha$  levels since  $\Pr(>|t|)$ , the  $p$  value is (for all intents and purposes) zero.
- A significant  $p$  value for this intercept implies that the predicted recovery score for a patient fed a popsicle with 0 mEq/l of the electrolytic solution will be different than 0%.
- By running the `confint` function we have previously seen, we can establish a confidence interval for the intercept term (and the slope of dose, as well).

```
confint(lm(recov.score ~ dose, data = hydrate), level = .95)
```

	2.5 %	97.5 %
(Intercept)	55.827	72.0
dose	0.466	9.3

The t test for the slope of `dose`, on the other hand, is important. This tests the hypothesis that the true slope of `dose` is zero vs. a two-tailed alternative.

If the slope of dose was in fact zero, then this would mean that knowing the dose information would be of no additional value in predicting the outcome over just guessing the mean of `recov.score` for every subject.

So, since the slope of dose is significantly different than zero (as it is at the 5% significance level, since  $p = 0.031$ ),

- `dose` has statistically significant predictive value for `recov.score`,
- more generally, this model has statistically significant predictive value as compared to a model that ignores the `dose` information and simply predicts the mean of `recov.score` for each subject.

### 40.3.6 Summarizing the Quality of Fit

4. The next part of the regression summary output is a summary of fit quality.

The **residual standard error** estimates the standard deviation of the prediction errors made by the model.

- If assumptions hold, the model will produce residuals that follow a Normal distribution with mean 0 and standard deviation equal to this residual standard error.

- So we'd expect roughly 95% of our residuals to fall between  $-2(12.21)$  and  $+2(12.21)$ , or roughly  $-24.4$  to  $+24.4$  and that we'd see virtually no residuals outside the range of  $-3(12.21)$  to  $+3(12.21)$ , or roughly  $-36.6$  to  $+36.6$ .
- The output at the top of the summary tells us about the observed regression residuals, and that they actually range from  $-22$  to  $+24$ .
- In context, it's hard to know whether or not we should be happy about this. On a scale from 0 to 100, rarely missing by more than 24 seems OK to me, but not terrific.
- The **degrees of freedom** here are the same as the denominator degrees of freedom in the ANOVA to follow. The calculation is  $n - k$ , where  $n$  = the number of observations and  $k$  is the number of coefficients estimated by the regression (including the intercept and any slopes).
  - Here, there are 36 observations in the model, and we fit  $k = 2$  coefficients; the slope and the intercept, as in any simple regression model, so  $df = 36 - 2 = 34$ .

The multiple  $R^2$  value is usually just referred to as  $R^2$  or R-squared.

- This is interpreted as the proportion of variation in the outcome variable that has been accounted for by our regression model.
  - Here, we've accounted for just under 13% of the variation in % Recovery using Dose.
- The  $R$  in multiple  $R$ -squared is the Pearson correlation of `recov.score` and `dose`, which in this case is 0.3595.
  - Squaring this value gives the  $R^2$  for this simple regression.
  - $(0.3595)^2 = 0.129$

$R^2$  is greedy.

- $R^2$  will always suggest that we make our models as big as possible, often including variables of dubious predictive value.
- As a result, there are various methods for adjusting or penalizing  $R^2$  so that we wind up with smaller models.
- The **adjusted  $R^2$**  is often a useful way to compare multiple models for the same response.
  - $R_{adj}^2 = 1 - \frac{(1-R^2)(n-1)}{n-k}$ , where  $n$  = the number of observations and  $k$  is the number of coefficients estimated by the regression (including the intercept and any slopes).
  - So, in this case,  $R_{adj}^2 = 1 - \frac{(1-0.1293)(35)}{34} = 0.1037$
  - The adjusted  $R^2$  value is not, technically, a proportion of anything, but it is comparable across models for the same outcome.
  - The adjusted  $R^2$  will always be less than the (unadjusted)  $R^2$ .

#### 40.3.7 ANOVA F test

5. The last part of the standard summary of a regression model is the overall ANOVA F test.

The hypotheses for this test are:

- $H_0$ : The model has **no** statistically significant predictive value, at all vs.
- $H_A$ : The model has statistically significant predictive value.

This is equivalent to:

- $H_0$ : Each of the coefficients in the model (other than the intercept) has  $\beta = 0$  vs.
- $H_A$ : At least one regression slope has  $\beta \neq 0$

Since we are doing a simple regression with just one predictor, the ANOVA F test hypotheses are exactly the same as the t test for dose:

- $H_0$ : The slope for `dose` has  $\beta = 0$  vs.
- $H_A$ : The slope for `dose` has  $\beta \neq 0$

In this case, we have an F statistic of 5.05 on 1 and 34 degrees of freedom, yielding  $p = 0.03$

- At  $\alpha = 0.05$ , we conclude that there is statistically significant predictive value somewhere in this model, since  $p < 0.05$ .
  - This is conclusive evidence that “something” in our model (here, `dose` is the only predictor) predicts the outcome to a degree beyond that easily attributed to chance alone.
- Another appropriate conclusion is that the  $R^2$  value (13%) is a statistically significant amount of variation in `recov.score` that is accounted for by a linear regression on `dose`.
- In *simple regression* (regression with only one predictor), the t test for the slope (`dose`) always provides the same p value as the ANOVA F test.
  - The F test statistic in a *simple regression* is always by definition just the square of the slope’s t test statistic.
  - Here,  $F = 5.047$ , and this is the square of  $t = 2.247$  from the Coefficients output

## 40.4 Viewing the complete ANOVA table

We can obtain the complete ANOVA table associated with this particular model, and the details behind this F test using the `anova` function:

```
anova(lm(recov.score ~ dose, data = hydrate))
```

Analysis of Variance Table

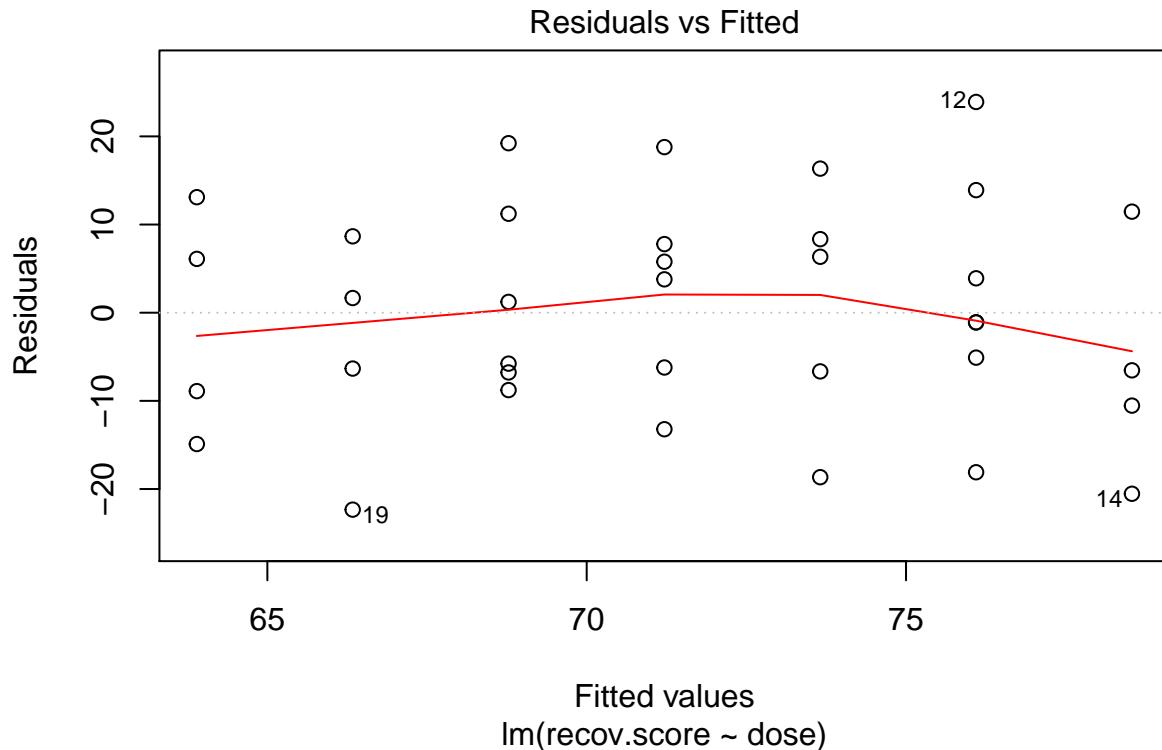
```
Response: recov.score
          Df Sum Sq Mean Sq F value Pr(>F)
dose       1    752     752   5.05   0.031 *
Residuals 34   5067     149
---
Signif. codes:  0 '***' 0.001 '**' 0.01 '*' 0.05 '.' 0.1 ' ' 1
```

- The  $R^2$  for our regression model is equal to the  $\eta^2$  for this ANOVA model.
  - If we divide  $SS(\text{dose}) = 752.2$  by the total sum of squares ( $752.2 + 5066.7$ ), we’ll get the multiple  $R^2 [0.1293]$
- Note that this is *not* the same ANOVA model we would get if we treated `dose` as a factor with seven levels, rather than as a quantitative variable.

## 40.5 Plotting Residuals vs. Fitted Values

We can obtain the plot of residuals vs. fitted values from this model using:

```
plot(lm(recov.score ~ dose, data = hydrate), which = 1)
```



We hope in this plot to see a generally random scatter of points, perhaps looking like a “fuzzy football”. Since we only have seven possible `dose` values, we obtain only seven distinct predicted values, which explains the seven vertical lines in the plot. Here, the smooth red line indicates a gentle curve, but no evidence of a strong curve, or any other regular pattern in this residual plot.

To save the residuals and predicted (fitted) values from this simple regression model, we can use the `resid` and `fitted` commands, respectively, or we can use the `augment` function in the `broom` package to obtain a tidy data set containing these objects and others.

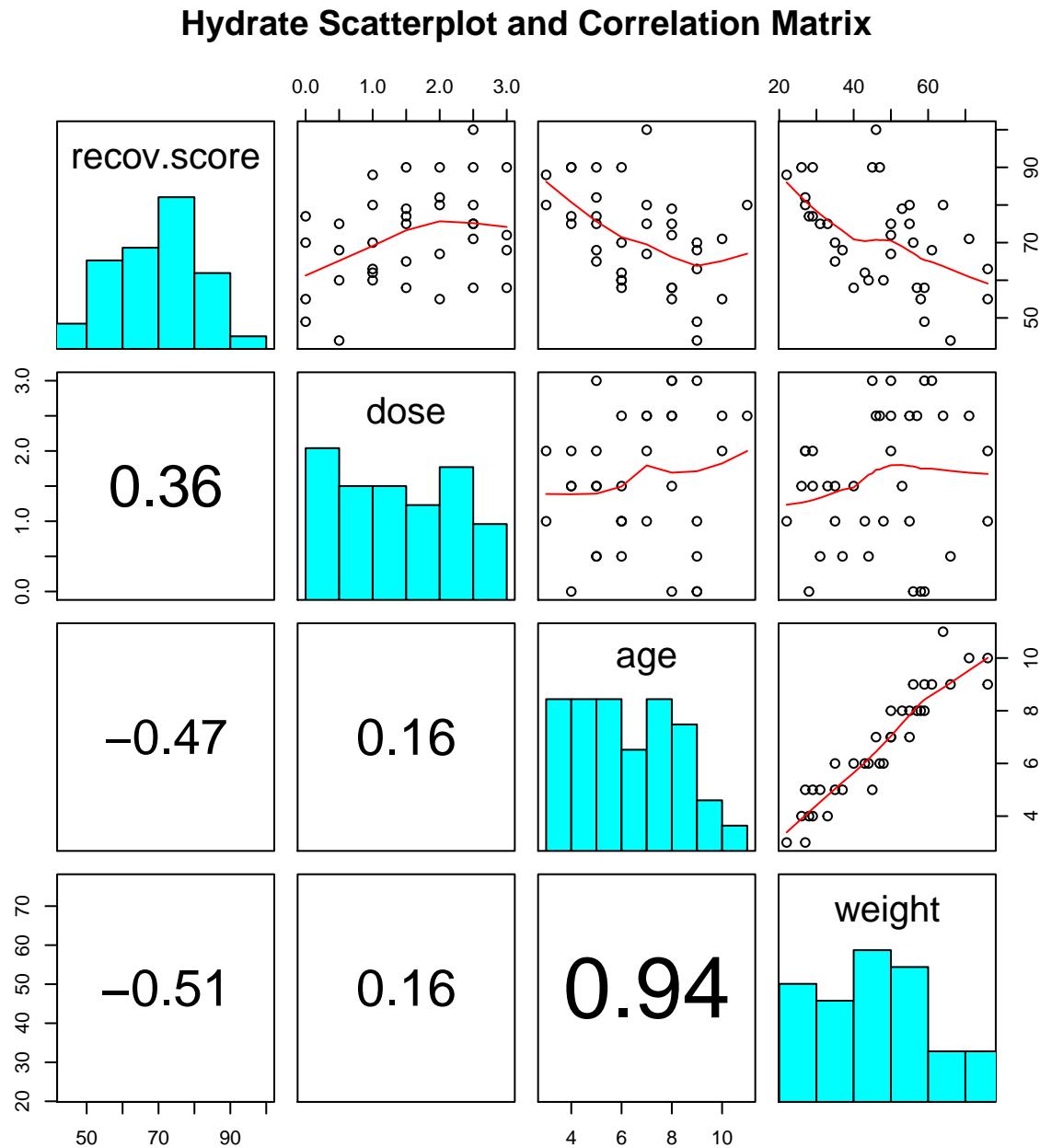
# Chapter 41

## Multiple Regression with the `hydrate` data

### 41.1 Another Scatterplot Matrix for the `hydrate` data

Along the diagonals of the scatterplot matrix, we have histograms of each of the variables. In the first row of the matrix, we have scatterplots of Recovery (on the y-axis) against each of the predictor variables in turn. We see a positive relationship with Dose, and a negative relationship with both Age, and then Weight. All possible scatterplots are shown, including plots that look at the association between the predictor variables.

```
pairs (~ recov.score + dose + age + weight, data=hydrate,
       main="Hydrate Scatterplot and Correlation Matrix",
       upper.panel = panel.smooth,
       diag.panel = panel.hist,
       lower.panel = panel.cor)
```



We see the positive correlation between Recovery and Dose (+.36) and the negative correlations between Recovery and Age (-.47) and Recovery and Weight (-.51). We can also see a very strong positive correlation between Weight and Age (+.94), which implies that it may be very difficult to separate out the effect of Weight from the effect of Age on our response.

## 41.2 A Multiple Regression for `recov.score`

Our first multiple linear regression model will predict `recov.score` using three predictors: `dose`, `age` and `weight`.

```
summary(lm(recov.score ~ dose + age + weight, data = hydrate))
```

Call:  
`lm(formula = recov.score ~ dose + age + weight, data = hydrate)`

Residuals:

Min	1Q	Median	3Q	Max
-16.68	-6.49	-2.20	7.67	22.13

Coefficients:

	Estimate	Std. Error	t value	Pr(> t )
(Intercept)	85.476	5.965	14.33	1.8e-15 ***
dose	6.170	1.791	3.45	0.0016 **
age	0.277	2.285	0.12	0.9043
weight	-0.543	0.324	-1.68	0.1032

---

Signif. codes: 0 '\*\*\*' 0.001 '\*\*' 0.01 '\*' 0.05 '.' 0.1 ' ' 1

Residual standard error: 9.92 on 32 degrees of freedom  
 Multiple R-squared: 0.458, Adjusted R-squared: 0.408  
 F-statistic: 9.03 on 3 and 32 DF, p-value: 0.000177

### 41.2.1 Model Specification

Call:  
`lm(formula = recov.score ~ dose + age + weight)`

The output begins by presenting the R function call. Here, we have a linear model, with `recov.score` being predicted using `dose`, `age` and `weight`, all from the `hydrate` data.

### 41.2.2 Model Residuals

Residuals:

Min	1Q	Median	3Q	Max
-16.682	-6.492	-2.204	7.667	22.128

Next, we summarize the residuals, where Residual = Actual Value - Predicted Value.

- This gives us a sense of how incorrect our predictions are for the `hydrate` observations.
  - The residuals will center near 0 (least squares is designed so the mean will always be zero, here the median prediction is 2.2 points too high)
  - Here, we predicted a `recov.score` that was 16.68 points too high for one patient, and another of our predicted `recov.score` values was 22.13 points too low.
  - The middle half of our predictions were between 6.5 points too low and 7.7 points too high.

### 41.2.3 Model Coefficients

Coefficients:

	Estimate	Std. Error	t value	Pr(> t )
(Intercept)	85.4764	5.9653	14.329	1.79e-15 ***
dose	6.1697	1.7908	3.445	0.00161 **
age	0.2770	2.2847	0.121	0.90428

```

weight      -0.5428     0.3236  -1.677  0.10325
---
Signif. codes:  0 '***' 0.001 '**' 0.01 '*' 0.05 '.' 0.1 ' ' 1

```

The first column of this table gives the estimated coefficients for our model (including the intercept, and slopes for each of our three predictors). Our least squares equation is `recov.score` = 85.48 + 6.17 `dose` + 0.28 `age` - 0.54 `weight`.

#### 41.2.4 Interpreting the t tests (last predictor in)

Next to each coefficient is its estimated standard error, followed by the coefficient's t value (the coefficient value divided by the standard error), and the associated two-tailed *p* value. The *p* value addresses  $H_0$ : This coefficient's  $\beta$  value = 0, as **last predictor into the model**.

- The t test of the intercept is rarely of interest, because
  - a. we rarely care about the situation that the intercept predicts; where all of the predictor variables are equal to zero, and
  - b. we usually are going to keep the intercept in the model regardless of its statistical significance.

The t test for dose tests the hypothesis that the slope of dose should be zero, as **last predictor in**.

- If the slope of `dose` was in fact zero, that would mean that knowing the `dose` information would be of no additional value in predicting the outcome after you have accounted for `age` and `weight` in your model.
- **Last predictor in**
  - This **last predictor in** business means that the test is comparing a model with `dose`, `age` and `weight` to a model with `age` and `weight` alone, to see if the incremental benefit of adding `dose` provides statistically significant additional predictive value for `recov.score`.
  - The t test tells us if `dose` is a useful addition to a model that already contains the other two variables.
  - The *p* value = .0016, so, at any reasonable  $\alpha$  level, the `dose` received is a significant part of the predictive model, even after accounting for `age` and `weight`.

##### 41.2.4.1 The t test of `age`

The t test of `age` tests  $H_0$ : `age` has no predictive value for `recov.score` as last predictor in.

- Here *p* = .9043, which indicates that `age` doesn't add statistically significant predictive value to the model once we have adjusted for `dose` and `weight`.
- This **does not** mean that `age`, by itself, has no linear relationship with `recov.score`, but it does mean that in this model, it doesn't help to add `age` once we already have `dose` and `weight`.
- This shouldn't be surprising, given the strong correlation between `age` and `weight` shown in the scatterplot matrix.

##### 41.2.4.2 The t test of `weight`

The t test of `weight` tests the null hypothesis that `weight` has no predictive value for `recov.score` as last predictor in.

- Here *p* = .1032, which is better than we saw in the `age` variable, but still indicates that `weight` adds no significant predictive value to the model for `recov.score` if we already have `dose` and `age`.

### 41.2.5 The Effect of Collinearity

The situation we see here with `age` and `weight`, where two predictors are highly correlated with one another, making it hard to see significant predictive value for either one of them after the other is already in the model is referred to as **collinearity**.

- Our usual approach to dealing with this collinearity will be to consider dropping one predictor from our model, and refit.
- Dropping either predictor will likely have a fairly small impact on the fit quality of our model, but if we drop both, we may do a lot of damage.

### 41.2.6 Confidence Intervals for the Slopes in a Multiple Regression Model

Here are the confidence intervals for the coefficients of our multiple regression model.

```
confint(lm(recov.score ~ dose + age + weight, data = hydrate))
```

	2.5 %	97.5 %
(Intercept)	73.33	97.627
dose	2.52	9.817
age	-4.38	4.931
weight	-1.20	0.116

We conclude, for instance, with 95% confidence, that that true slope of `dose` is between 2.52 and 9.82 points on the recovery score scale.

This is pretty different from the confidence interval we found for the slope of `dose` in a simple regression on `dose` alone that we saw previously, and repeat below.

```
confint(lm(recov.score ~ dose, data = hydrate), level = .95)
```

	2.5 %	97.5 %
(Intercept)	55.827	72.0
dose	0.466	9.3

In both cases, the reasonable range of values for the slope of `dose` appears to be positive, but the range of values is much tighter in the multiple regression.

### 41.2.7 Model Summaries

```
Residual standard error: 9.923 on 32 degrees of freedom
Multiple R-squared:  0.4584,    Adjusted R-squared:  0.4077
F-statistic:  9.03 on 3 and 32 DF,  p-value: 0.0001769
```

The **residual standard error** estimates the standard deviation of our model's errors to be 9.923.

- We'd expect roughly 95% of our residuals to fall between  $-2(9.923)$  and  $+2(9.923)$ , or roughly -20 to +20.
- We'd expect to see virtually no residuals outside the range of  $\pm 3(9.923)$  or roughly -30 to +30.

The **coefficient of determination**,  $R^2$ , is estimated to be 0.4584

- We accounted for just under 46% of the variation in `recov.score` using `dose`, `age` and `weight`.

$R^2$  will always suggest that we make our models as big as possible, often including variables of dubious predictive value.

- An important thing to realize is that **if you add a variable to a model,  $R^2$  cannot decrease**.

- Similarly, removing a variable from a model, no matter how unrelated to the outcome, cannot increase the  $R^2$  value.
- Thus, using  $R^2$  as the be-all and end-all of model fit for a regression model goes against the general notion that we would like to have parsimonious models; that is to say, we favor simple models when possible.

The overall ANOVA F test is presented last.

- The test gives an F-statistic of 9.03 on 3 and 32 degrees of freedom, yielding a p value of .00018.
- We can conclude (at any reasonable  $\alpha$  level) that there is statistically significant predictive value somewhere in this model.
- The F test for a multiple regression is a very low standard; specifying only that we have conclusive evidence that some part of the model predicts the outcome to a degree beyond that easily attributed to chance alone.
- We return to the individual t tests to assess significance after adjusting for the other predictors.
- Concluding that the F test is significant means that the multiple  $R^2$  accounted for by the model is also statistically significant.

#### 41.2.7.1 Verifying the $R^2$ Calculations

The formula for  $R^2$  is simply the sum of squares accounted for by the regression model divided by the total sum of squares.

- This is the same as 1 minus [residual sum of squares divided by total sum of squares].
- We can verify the calculation with the complete ANOVA table for this model

```
anova(lm(recov.score ~ dose + weight + age, data = hydrate))
```

Analysis of Variance Table

```
Response: recov.score
          Df Sum Sq Mean Sq F value    Pr(>F)
dose       1    752     752   7.64 0.00940 ***
weight     1   1914    1914  19.44 0.00011 ***
age        1      1      1   0.01 0.90428
Residuals 32   3151     98
---
```

Signif. codes: 0 '\*\*\*' 0.001 '\*\*' 0.01 '\*' 0.05 '.' 0.1 ' ' 1

$$\text{So } R^2 = 1 - \frac{SS(\text{Residual})}{SS(\text{Total})} = 1 - \frac{3151.22}{752.15+1914.07+1.45+3151.22} = 1 - \frac{3151.22}{5818.89} = 1 - 0.5416 = .4584$$

If we are fitting a model to  $n$  data points, and we have  $k$  coefficients (slopes + intercept) in our model, then the model's adjusted  $R^2$  is  $R_{adj}^2 = 1 - \frac{(1-R^2)(n-1)}{n-k}$ .

In the `hydrate` data, we have  $n = 36$  data points, and  $k = 4$  coefficients (3 slopes, plus the intercept), so  $R_{adj}^2 = 1 - \frac{(1-.4584)(36-1)}{36-4} = .4077$

- Again, the adjusted  $R^2$  is *not* interpreted as a percentage of anything, and can in fact be negative.
- $R_{adj}^2$  will always be less than the original  $R^2$  so long as there is at least one predictor besides the intercept term, so that  $k > 1$  in the equation above.

## 41.3 ANOVA for Sequential Comparison of Models

Outside of the main summary, we can also run a sequence of comparisons of the impact of various predictors in our model with the `anova` function.

```
anova(lm(recov.score ~ dose + age + weight, data = hydrate))
```

Analysis of Variance Table

```
Response: recov.score
          Df Sum Sq Mean Sq F value Pr(>F)
dose      1    752     752   7.64 0.00940 **
age       1   1639    1639  16.64 0.00028 ***
weight    1    277     277   2.81 0.10325
Residuals 32   3151     98
---
Signif. codes:  0 '***' 0.001 '**' 0.01 '*' 0.05 '.' 0.1 ' ' 1
```

This ANOVA table is very different from the one we saw in our simple regression model. The various  $p$  values shown here indicate the significance of predictors taken in turn, specifically...

1. The  $p$  value for  $H_0$ : `dose` has significant predictive value by itself is 0.009
2. The  $p$  value for  $H_0$ : `age` adds significant predictive value once you already have `dose` in the model is 0.0003
3. The  $p$  value for  $H_0$ : `weight` adds significant predictive value once you already have `dose` and `age` in the model (i.e. as last predictor in) is 0.1032

Note that this last  $p$  value is the same as the  $t$  test for weight we have already seen in the main summary of the model.

If we change the order of the predictors entering the model, the main summary of our linear model will not change, but these ANOVA results will change.

```
summary(lm(recov.score ~ dose + weight + age, data = hydrate))
```

Call:

```
lm(formula = recov.score ~ dose + weight + age, data = hydrate)
```

Residuals:

Min	1Q	Median	3Q	Max
-16.68	-6.49	-2.20	7.67	22.13

Coefficients:

	Estimate	Std. Error	t value	Pr(> t )
(Intercept)	85.476	5.965	14.33	1.8e-15 ***
dose	6.170	1.791	3.45	0.0016 **
weight	-0.543	0.324	-1.68	0.1032
age	0.277	2.285	0.12	0.9043

```
---
Signif. codes:  0 '***' 0.001 '**' 0.01 '*' 0.05 '.' 0.1 ' ' 1
```

Residual standard error: 9.92 on 32 degrees of freedom

Multiple R-squared: 0.458, Adjusted R-squared: 0.408

F-statistic: 9.03 on 3 and 32 DF, p-value: 0.000177

```
anova(lm(recov.score ~ dose + weight + age, data = hydrate))
```

Analysis of Variance Table

```
Response: recov.score
          Df Sum Sq Mean Sq F value Pr(>F)
```

```

dose      1    752     752   7.64 0.00940 ***
weight     1   1914    1914  19.44 0.00011 ***
age        1       1      1   0.01 0.90428
Residuals 32   3151     98
---
Signif. codes:  0 '***' 0.001 '**' 0.01 '*' 0.05 '.' 0.1 ' ' 1

```

- Does `age` add significant predictive value to the model including `dose` and `weight`?
  - **No**, because the  $p$  value for `age` is 0.904 once we have previously accounted for `dose` and `weight`.
- Does `weight` add significant predictive value to the model that includes `dose` only?
  - **Yes**, because the  $p$  value for `weight` is 0.00011 once we have accounted for `dose`.

### 41.3.1 Building The ANOVA Table for The Model

Sometimes, we want an ANOVA table for the Regression as a whole, to compare directly to the Residuals. We can build this by adding up the sums of squares and degrees of freedom from the individual predictors, then calculating the Mean Square and F ratio for the Regression.

- The degrees of freedom are easy. We have three predictors (slopes), each accounting for one DF, and we have 32 DF applied to the residuals, so total DF =  $n - 1 = 35$

ANOVA Table	DF	SS	MS	F	$p$ value
Regression	3	?	?	?	?
Residuals	32	3151.22	98.48		
Total	35	?			

- To obtain the sum of squares due to the regression model, we just add the sums of squares for the individual predictors, so SS(Regression) =  $752.15 + 1914.07 + 1.45 = 2667.67$
- The total sum of squares is then the sum of SS(Regression) and SS(Residuals): here,  $2667.67 + 3151.22 = 5818.89$
- Now, we recall that the Mean Square for any row in this table is just the Sum of Squares divided by the degrees of freedom, so that MS(Regression) =  $2667.67 / 3 = 889.22$

ANOVA Table	DF	SS	MS	F	$p$ value
Regression	3	2667.67	889.22	?	?
Residuals	32	3151.22	98.48		
Total	35	5818.89			

- The F ratio and p value can be obtained from the original summary of the model.

ANOVA Table	DF	SS	MS	F	$p$ value
Regression	3	2667.67	889.22	9.03	0.00018
Residuals	32	3151.22	98.48		
Total	35	5818.89			

We can use this to verify that the  $R^2$  for the model is also equal to  $\text{SS}(\text{Regression}) / \text{SS}(\text{Total})$

$$R^2 = \frac{\text{SS}(\text{Regression})}{\text{SS}(\text{Total})} = \frac{2667.67}{5818.89} = 0.4584$$

## 41.4 Standardizing the Coefficients of a Model

Which of the three predictors: `dose`, `age` and `weight`, in our model for `recov.score`, has the largest effect?

Sometimes, we want to express the coefficients of the regression in a standardized way, to compare the impact of each predictor within the model on a fairer scale. A common trick is to “standardize” each input variable (predictor) by subtracting its mean and dividing by its standard deviation. Each coefficient in this semi-standardized model is the expected difference in the outcome, comparing subjects that differ by one standard deviation in one variable with all other variables fixed at their average. R can do this rescaling quite efficiently, with the use of the `scale` function.

### 41.4.1 A Semi-Standardized Model for the `hydrate` data

```
summary(lm(recov.score ~ scale(dose) + scale(weight) + scale(age), data = hydrate))
```

Call:

```
lm(formula = recov.score ~ scale(dose) + scale(weight) + scale(age),
  data = hydrate)
```

Residuals:

Min	1Q	Median	3Q	Max
-16.68	-6.49	-2.20	7.67	22.13

Coefficients:

	Estimate	Std. Error	t value	Pr(> t )
(Intercept)	71.556	1.654	43.26	<2e-16 ***
scale(dose)	5.860	1.701	3.45	0.0016 **
scale(weight)	-8.040	4.794	-1.68	0.1032
scale(age)	0.581	4.793	0.12	0.9043

---

Signif. codes: 0 '\*\*\*' 0.001 '\*\*' 0.01 '\*' 0.05 '.' 0.1 ' ' 1

Residual standard error: 9.92 on 32 degrees of freedom

Multiple R-squared: 0.458, Adjusted R-squared: 0.408

F-statistic: 9.03 on 3 and 32 DF, p-value: 0.000177

The only things that change here are the estimates and standard errors of the coefficients: every other bit of the output is unchanged from our original summary.

- Each of the scaled covariates has mean zero and standard deviation one.
  - `scale(dose)`, for instance, is obtained by subtracting the mean from the original `dose` variable (so the result is centered at zero) and dividing that by the standard deviation (so that `scale(dose)` has mean 0 and standard deviation 1.)
  - We interpret the coefficient of `scale(dose) = 5.86` as the change in our outcome (`recov.score`) that we anticipate when the dose increases by one standard deviation from its mean, while all of the other inputs (`weight` and `age`, specifically) remain at their means.

This allows us to compare the effects on `recov.score` due to `dose`, to `weight` and to `age` in terms of a change of one standard deviation in each, while holding the others constant.

- Which of the inputs appears to have the biggest impact on recovery score in this sense?

#### 41.4.1.1 Interpreting the Intercept in a Model with Semi-Standardized Coefficients

The semi-standardized model has an interesting intercept term. The intercept is equal to the mean of our outcome (`recov.score`) across the full set of subjects.

- When `scale(dose)`, `scale(weight)` and `scale(age)` are all zero, this means that `dose`, `weight` and `age` are at their means.
- So the intercept tells you the value of `recov.score` that we would predict when all of the scaled predictors are zero, e.g., when all of the original inputs to the model (`dose`, `weight` and `age`) are at their average values.

## 41.5 Comparing Fits of Several Possible Models for Recovery Score

Below, I summarize the results for five possible models for `recov.score`. What can we conclude? By this set of results, which of the models looks best?

Model	Fitted Equation	R <sup>2</sup>	R <sup>2</sup> <sub>adj</sub>	RSE	F test p
[Int]	71.6	-	-	12.89	-
D	63.9 + 4.88 dose	.1293	.1037	12.21	0.0313
DA	84.1 + 6.07 dose - 3.31 age	.4108	.3751	10.19	0.0002
DW	85.6 + 6.18 dose - 0.51 weight	.4582	.4254	9.77	4.1e-05
DAW	85.5 + 6.17 dose + 0.28 age - 0.54 weight	.4584	.4077	9.92	0.0002

It appears as though the DW model is almost as good as the DAW model using the multiple R<sup>2</sup> as a criterion, and is the best of these five models using any of the other criteria.

The five summaries I used to obtain this table were obtained with the following code (not evaluated here):

```
summary(lm(recov.score ~ 1))
summary(lm(recov.score ~ dose))
summary(lm(recov.score ~ dose + age))
summary(lm(recov.score ~ dose + weight))
summary(lm(recov.score ~ dose + age + weight))
```

## 41.6 Comparing Model Fit: The AIC, or Akaike Information Criterion

Another summary we'll use to evaluate a series of potential regression models for the same outcome is the Akaike Information Criterion or AIC. Smaller values indicate better models, by this criterion, which is just a measure of relative quality.

Model	Intercept only	D	DA	DW	DAW
AIC	289.24	286.25	274.19	271.17	273.16

By the AIC, DW looks best out of these five models.

*Note:* R uses the `AIC` function applied to a model to derive these results. For example,

```
AIC(lm(recov.score ~ 1, data = hydrate))

[1] 289

AIC(lm(recov.score ~ dose + age + weight, data = hydrate))

[1] 273
```

## 41.7 Comparing Model Fit with the BIC, or Bayesian Information Criterion

Another summary we'll use to evaluate potential regression models for the same outcome is the Bayesian Information Criterion or BIC. Like the AIC, smaller values indicate better models, by this criterion, which is also a measure of relative quality.

Model	Intercept only	D	DA	DW	DAW
BIC	292.4	291.0	280.5	277.5	281.1

By the BIC, as well, DW looks best out of these five models.

To obtain the results, just apply BIC instead of AIC to the model.

```
BIC(lm(recov.score ~ dose + weight, data = hydrate))

[1] 278
```

## 41.8 Making Predictions for New Data: Prediction vs. Confidence Intervals

The `predict` function, like the `fitted` function, when applied to a linear model, can produce the fitted values predicted by the model. Yet there is more we can do with `predict`.

Suppose we want to use the model to predict our outcome (recovery score, or `recov.score`) on the basis of the three predictors (`dose`, `age` and `weight`.) Building an interval forecast around a fitted value requires us to decide whether we are:

- predicting the `recov.score` for **one particular child** with the specified characteristics (in which case we use something called a **prediction interval**) or
- predicting the mean `recov.score` across **all children** that have the specified `dose`, `age` and `weight` characteristics (in which case we use a **confidence interval**).

The *prediction* interval will always be wider than the related *confidence* interval.

### 41.8.1 Making a Prediction using the `hydrate` data

Now, suppose that we wish to predict a `recov.score` associated with `dose = 2`, `age = 7` and `weight = 50`. The approach I would use follows...

```
modela <- lm(recov.score ~ dose + age + weight, data = hydrate)
newdata <- data.frame(dose = 2, age = 7, weight = 50)
predict(modela, newdata, interval="prediction", level=0.95)
```

```

fit lwr upr
1 72.6 52.1 93.2

predict(modela, newdata, interval="confidence", level=0.95)

```

```

fit lwr upr
1 72.6 68.9 76.4

```

So, our conclusions are:

- If we have one particular child with `dose = 2`, `age = 7` and `weight = 50`, we have 95% confidence that the `recov.score` for this child will be between 52.1 and 93.2.
- If we have a large group of children with `dose = 2`, `age = 7` and `weight = 50`, we have 95% confidence that the average `recov.score` for these children will be between 68.9 and 76.4.

### 41.8.2 A New Prediction?

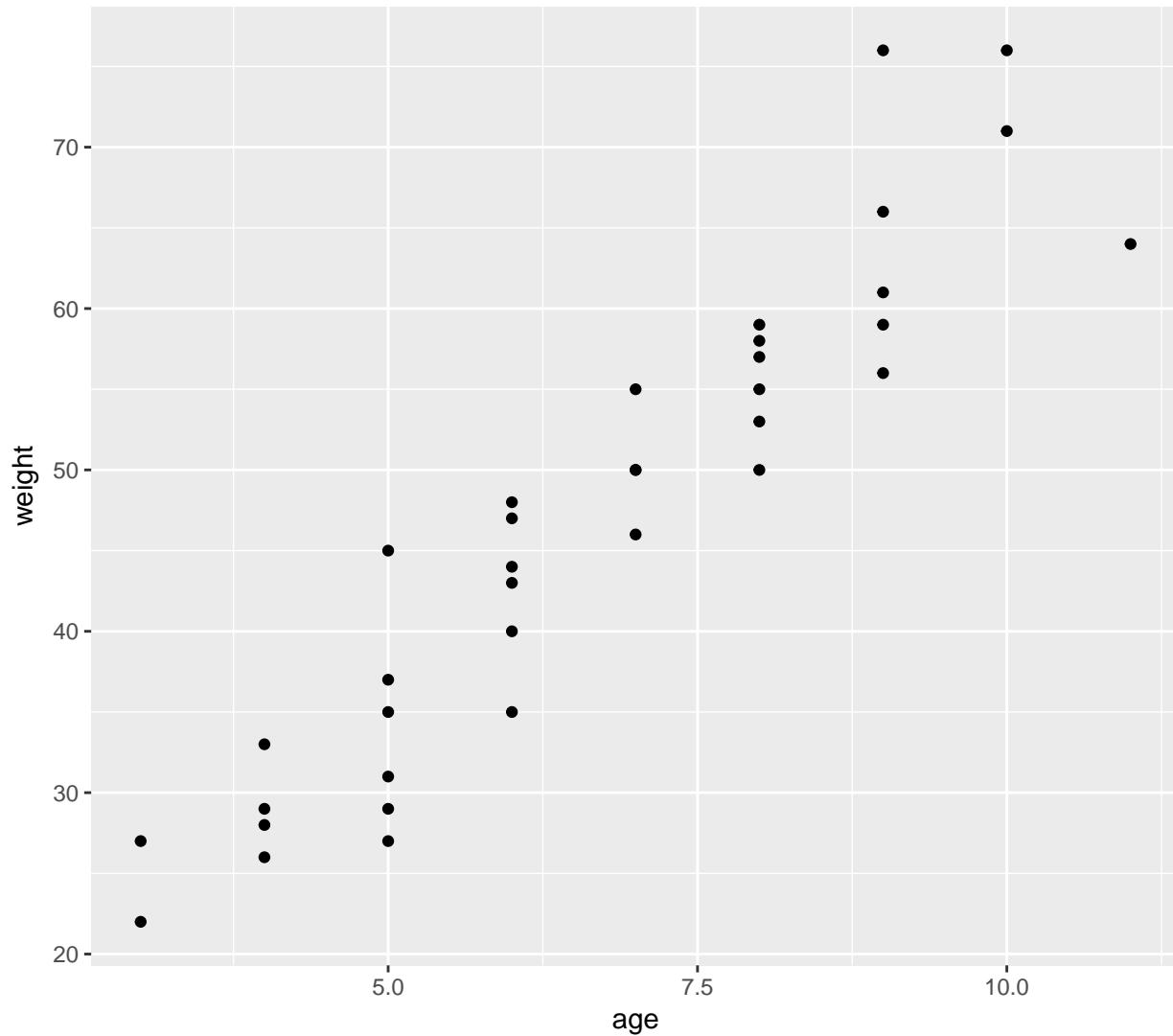
Suppose that now you wanted to make a prediction for a `recov.score` with `dose = 1`, `age = 4` and `weight = 60`. Why would this be a meaningfully worse idea than the prediction we just made, and how does the plot below tell us this?

```

ggplot(hydrate, aes(x = age, y = weight)) +
  geom_point() +
  labs(title = "Weight vs. Age in the hydrate data")

```

### Weight vs. Age in the hydrate data



## 41.9 Interpreting the Regression Model: Two Key Questions

Suppose we land, finally, on the DW model...

```
summary(lm(recov.score ~ dose + weight, data = hydrate))
```

```
Call:
lm(formula = recov.score ~ dose + weight, data = hydrate)
```

Residuals:

Min	1Q	Median	3Q	Max
-16.61	-6.49	-2.12	7.60	22.25

Coefficients:

Estimate	Std. Error	t value	Pr(> t )
----------	------------	---------	----------

```
(Intercept) 85.594      5.797    14.77  4.3e-16 ***
dose        6.175       1.763     3.50   0.0013 **
weight      -0.506     0.113    -4.48  8.6e-05 ***
---
Signif. codes:  0 '***' 0.001 '**' 0.01 '*' 0.05 '.' 0.1 ' ' 1
```

Residual standard error: 9.77 on 33 degrees of freedom

Multiple R-squared: 0.458, Adjusted R-squared: 0.425

F-statistic: 14 on 2 and 33 DF, p-value: 4.06e-05

Suppose we decide that this model is a reasonable choice, based on adherence to regression assumptions (as we'll discuss shortly), and quality of fit as measured both by  $R^2$  measures, the various hypothesis tests, and the information criteria (AIC and BIC).

**Question 1. Can we summarize the model and how well it fits the data in a reasonable English sentence or two?**

- Together, dose and weight account for just under 46% of the variation in recovery scores, and this is highly statistically significant ( $p < 0.001$ ). Higher recovery scores are associated with higher doses and with lower weights among the 36 children assessed in this study.

**Question 2. How might we interpret the coefficient of dose for someone who was smart but not a student of statistics?**

- If we have two kids who are the same weight, then if kid A receives a dose that is 1 mEq/L larger than kid B, we'd expect kid A's recovery score to be a little over 6 points better than the score for kid B.

## Chapter 42

# Regression Diagnostics

Some of this discussion comes from Bock, Velleman, and De Veaux (2004).

Multiple linear regression (also called ordinary least squares, or OLS) has four main assumptions that are derived from its model.

For a simple regression, the model underlying the regression line is  $E(Y) = \beta_0 + \beta_1 x$

- where  $E(Y)$  = the expectation (mean) of the outcome  $Y$ ,
- $\beta_0$  is the intercept, and
- $\beta_1$  is the slope

Now, if we have a multiple regression with three predictors  $x_1, x_2$  and  $x_3$ , as we do in the `hydrate` case (`dose`, `age` and `height`), then the model becomes  $E(Y) = \beta_0 + \beta_1 x_1 + \beta_2 x_2 + \beta_3 x_3$ .

- As the simple regression model did, the multiple regression model predicts the mean of our outcome, for a subject (or group of subjects) with specific values of the predictors.
- In the `hydrate` example, our model is  $E(\text{'recov.score'}) = \beta_0 + \beta_{\text{dose}} \text{dose} + \beta_{\text{age}} \text{age} + \beta_{\text{weight}} \text{weight}$ 
  - In a larger version of this study, we can imagine finding many kids with the same `age` and `weight` receiving the same `dose`, but they won't all have exactly the same `recov.score`.
  - Instead, we'd have many different recovery score values.
  - It is the mean of that distribution of recovery scores that the regression model predicts.

Alternatively, we can write the model to relate individual  $y$ 's to the  $x$ 's by adding an individual error term:  $y = \beta_0 + \beta_1 x_1 + \beta_2 x_2 + \beta_3 x_3 + \epsilon$

Of course, the multiple regression model is not limited to three predictors. We will often use  $k$  to represent the number of coefficients (slopes plus intercept) in the model, and will also use  $p$  to represent the number of predictors (usually  $p = k - 1$ ).

### 42.1 The Four Key Regression Assumptions

The assumptions and conditions for a multiple regression model are nearly the same as those for simple regression. The key assumptions that must be checked in a multiple regression model are:

- Linearity Assumption
- Independence Assumption
- Equal Variance (Constant Variance / Homoscedasticity) Assumption
- Normality Assumption

Happily, R is well suited to provide us with multiple diagnostic tools to generate plots and other summaries that will let us look more closely at each of these assumptions.

## 42.2 The Linearity Assumption

We are fitting a linear model.

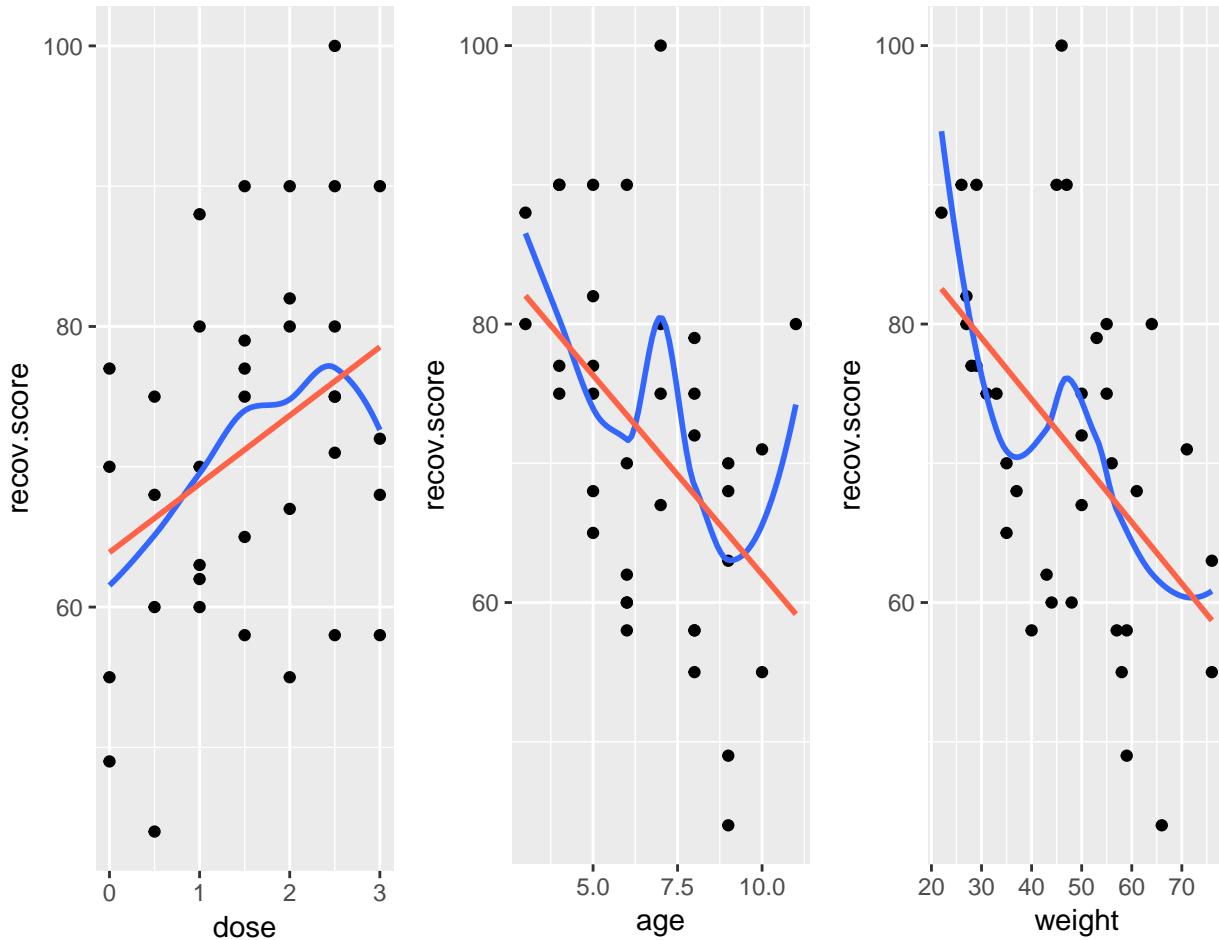
- By *linear*, we mean that each predictor value,  $x$ , appears simply multiplied by its coefficient and added to the model.
- No  $x$  appears in an exponent or some other more complicated function.
- If the regression model is true, then the outcome  $y$  is linearly related to each of the  $x$ 's.

Unfortunately, assuming the model is true is not sufficient to prove that the linear model fits, so we check what Bock, Velleman, and De Veaux (2004) call the “Straight Enough Condition”

### 42.2.1 Initial Scatterplots for the “Straight Enough” Condition

- Scatterplots of  $y$  against each of the predictors are reasonably straight.
- The scatterplots need not show a strong (or any!) slope; we just check that there isn't a bend or other nonlinearity.
- Any substantial curve is indicative of a potential problem.
- Modest bends are not usually worthy of serious attention.

For example, in the `hydrate` data, here are the relevant scatterplots (in practice, I would simply look at the scatterplot matrix produced earlier.)



Here, I've simply placed `recov.score` on the vertical (Y) axis, plotted against each of the predictors, in turn. I've added a straight line (OLS) fit [in tomato red] and a loess smooth [in blue] to each plot to guide your assessment of the “straight enough” condition.

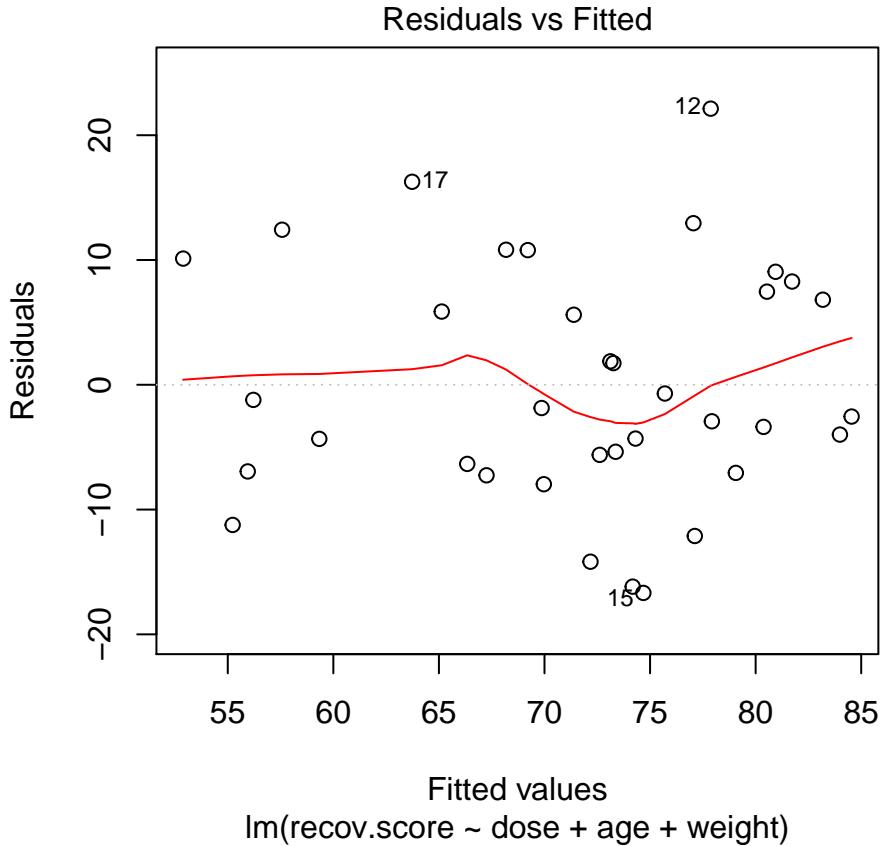
- Each of these is “straight enough” for our purposes, in initially fitting the data.
- If one of these was not, we might consider a transformation of the relevant predictor, or, if all were problematic, we might transform the outcome Y.

### 42.2.2 Residuals vs. Predicted Values to Check for Non-Linearity

The residuals should appear to have no pattern (no curve, for instance) with respect to the predicted (fitted) values. It is a very good idea to plot the residuals against the fitted values to check for patterns, especially bends or other indications of non-linearity. For a multiple regression, the fitted values are a combination of the x's given by the regression equation, so they combine the effects of the x's in a way that makes sense for our particular regression model. That makes them a good choice to plot against. We'll check for other things in this plot, as well.

When you ask R to plot the result of a linear model, it will produce up to five separate plots: the first of which is a plot of **residuals vs. fitted values**. To obtain this plot for the model including `dose`, `age` and `weight` that predicts `recov.score`, we indicate plot 1 using the `which` command within the `plot` function:

```
plot(lm(recov.score ~ dose + age + weight, data = hydrate), which=1)
```



The loess smooth is again added to help you identify serious non-linearity. In this case, I would conclude that there were no serious problems with linearity in these data.

The plot also, by default, identifies the three values<sup>1</sup> with the largest (in absolute value) residuals.

- Here, these are rows 12, 15 and 17, where 12 and 17 have positive residuals (i.e. they represent under-predictions by the model) and 15 has a negative residual (i.e. it represents a situation where the prediction was larger than the observed recovery score.)

### 42.2.3 Residuals vs. Predictors To Further Check for Non-Linearity

If we do see evidence of non-linearity in the plot of residuals against fitted values, I usually then proceed to look at residual plots against each of the individual predictors, in turn, to try to identify the specific predictor (or predictors) where we may need to use a transformation.

The appeal of such plots (as compared to the initial scatterplots we looked at of the outcome against each predictor) is that they eliminate the distraction of the linear fit, and let us look more closely at the non-linear part of the relationship between the outcome and the predictor.

Although I don't think you need them here, here are these plots for the residuals from this model.

```
hydrate$recov.res <- residuals(lm(recov.score ~ dose + age + weight, data = hydrate))
```

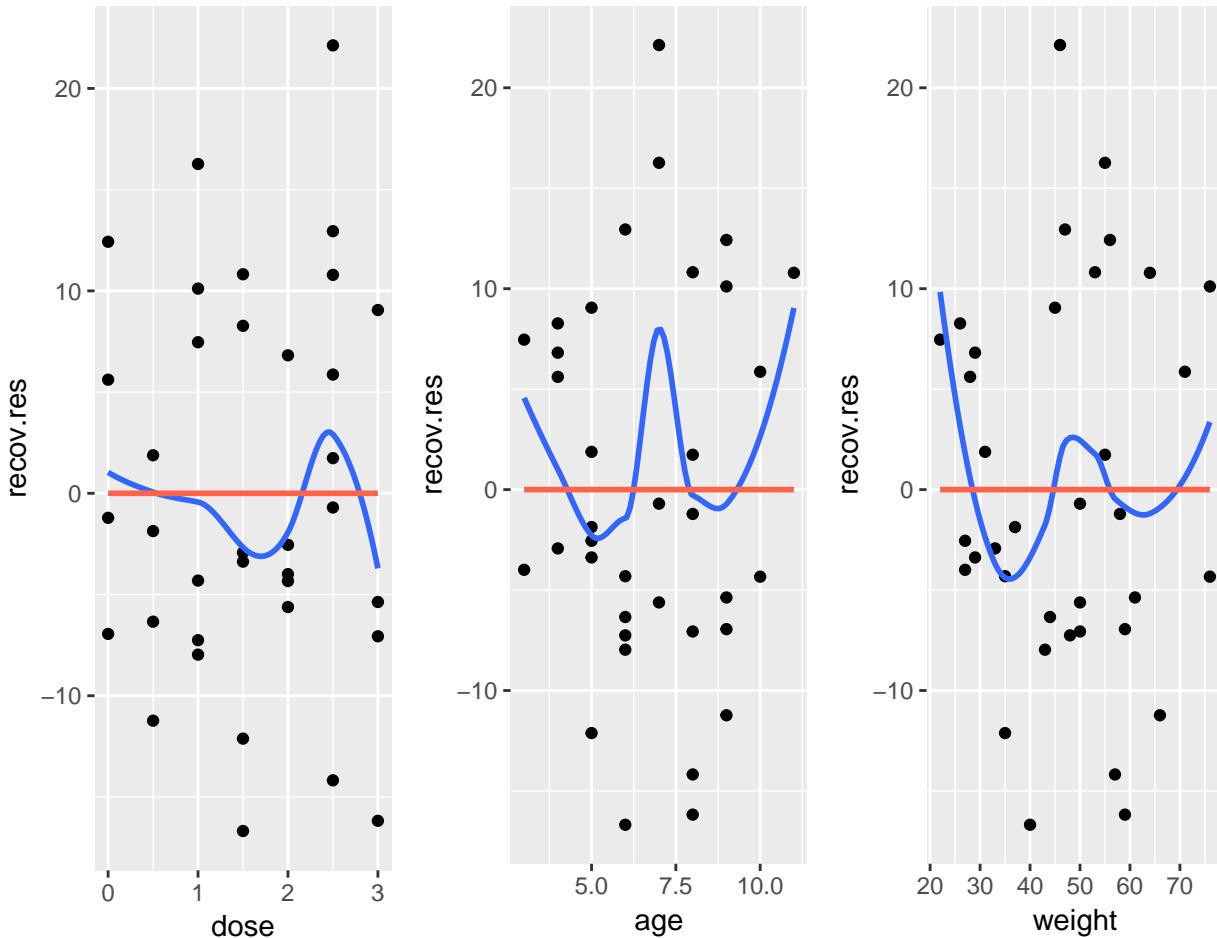
---

<sup>1</sup>If you wanted to identify more or fewer points, you could, i.e. ‘plot(modelname, which=1, id.n = 2)’

```

p1 <- ggplot(hydrate, aes(x = dose, y = recov.res)) +
  geom_point() + geom_smooth(method = "loess", se = FALSE) +
  geom_smooth(method = "lm", se = FALSE, col = "tomato")
p2 <- ggplot(hydrate, aes(x = age, y = recov.res)) +
  geom_point() + geom_smooth(method = "loess", se = FALSE) +
  geom_smooth(method = "lm", se = FALSE, col = "tomato")
p3 <- ggplot(hydrate, aes(x = weight, y = recov.res)) +
  geom_point() + geom_smooth(method = "loess", se = FALSE) +
  geom_smooth(method = "lm", se = FALSE, col = "tomato")
gridExtra::grid.arrange(p1, p2, p3, nrow=1)

```



Again, I see no particularly problematic issues in the scatterplot here. The curve in the `age` plot is a bit worrisome, but it still seems pretty modest, with the few values of 3 and 11 for `age` driving most of the “curved” pattern we see: there’s no major issue. If we’re willing to assume that the multiple regression model is correct in terms of its specification of a linear model, we can move on to assessing other assumptions and conditions.

## 42.3 The Independence Assumption

The errors in the true underlying regression model must be mutually independent, but there is no way to be sure that the independence assumption is true. Fortunately, although there can be many predictor variables,

there is only one outcome variable and only one set of errors. The independence assumption concerns the errors, so we check the residuals.

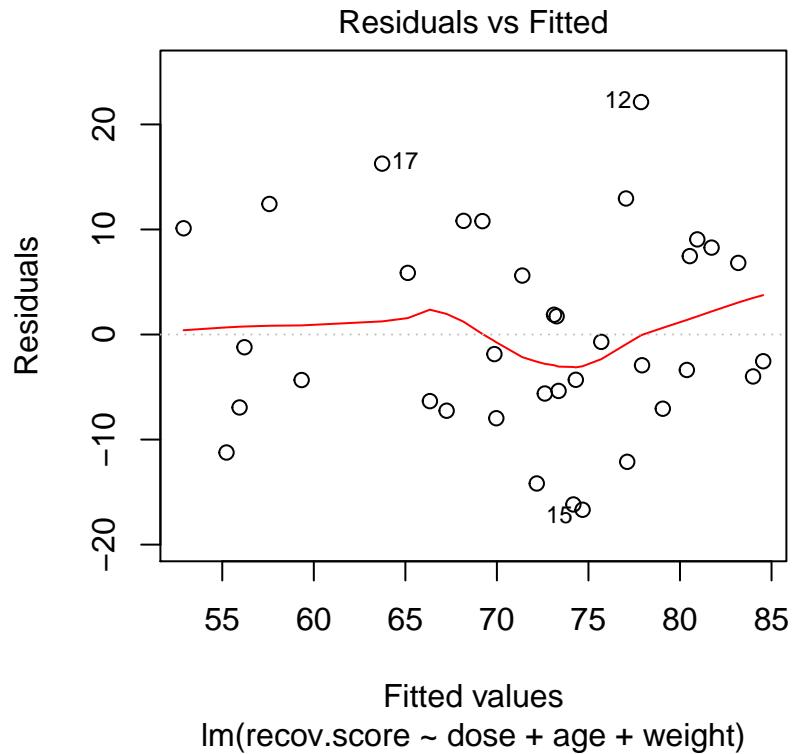
**Randomization condition.** The data should arise from a random sample, or from a randomized experiment.

- The residuals should appear to be randomly scattered and show no patterns, trends or clumps when plotted against the predicted values.
- In the special case when an x-variable is related to time, make sure that the residuals do not have a pattern when plotted against time.

### 42.3.1 Residuals vs. Fitted Values to Check for Dependence

The `hydrate` children were not related in any way and were randomly assigned to dosages, so we can be pretty sure that their measurements are independent. The residuals vs. fitted values plot shows no clear trend, cycle or pattern which concerns us.

```
plot(lm(recov.score ~ dose + age + weight, data = hydrate), which=1)
```



## 42.4 The Constant Variance Assumption

The variability of our outcome,  $y$ , should be about the same for all values of every predictor  $x$ . Of course, we can't check every combination of  $x$  values, so we look at scatterplots and check to see if the plot shows a "fan" shape - in essence, the **Does the plot thicken? Condition**.

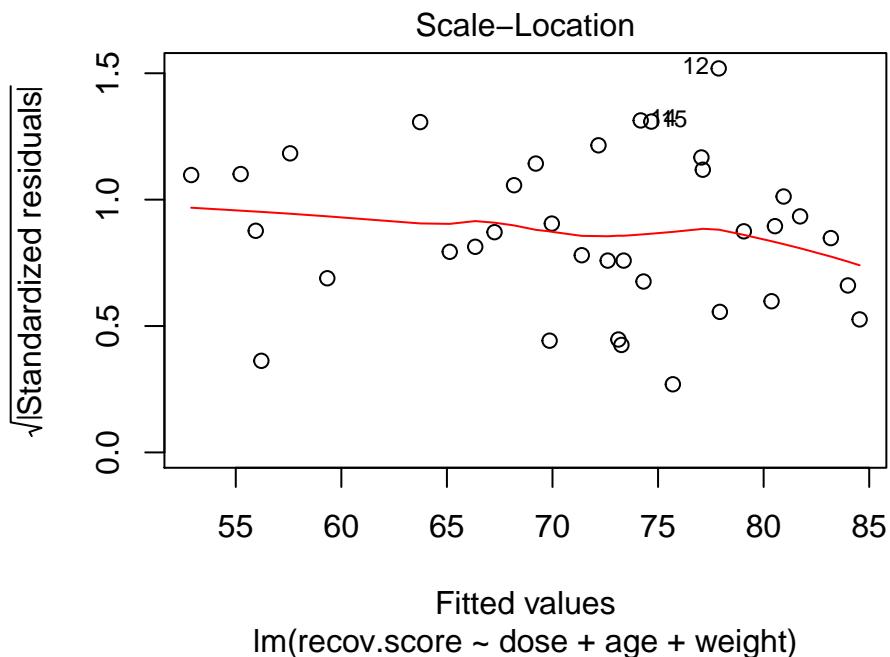
- Scatterplots of residuals against each  $x$  or against the predicted values, offer a visual check.
- Be alert for a "fan" or a "funnel" shape showing growing/shrinking variability in one part of the plot.

Reviewing the same plots we have previously seen, I can find no evidence of substantial “plot thickening” in either the plot of residuals vs. fitted values or in any of the plots of the residuals against each predictor separately.

#### 42.4.1 The Scale-Location Plot to Check for Non-Constant Variance

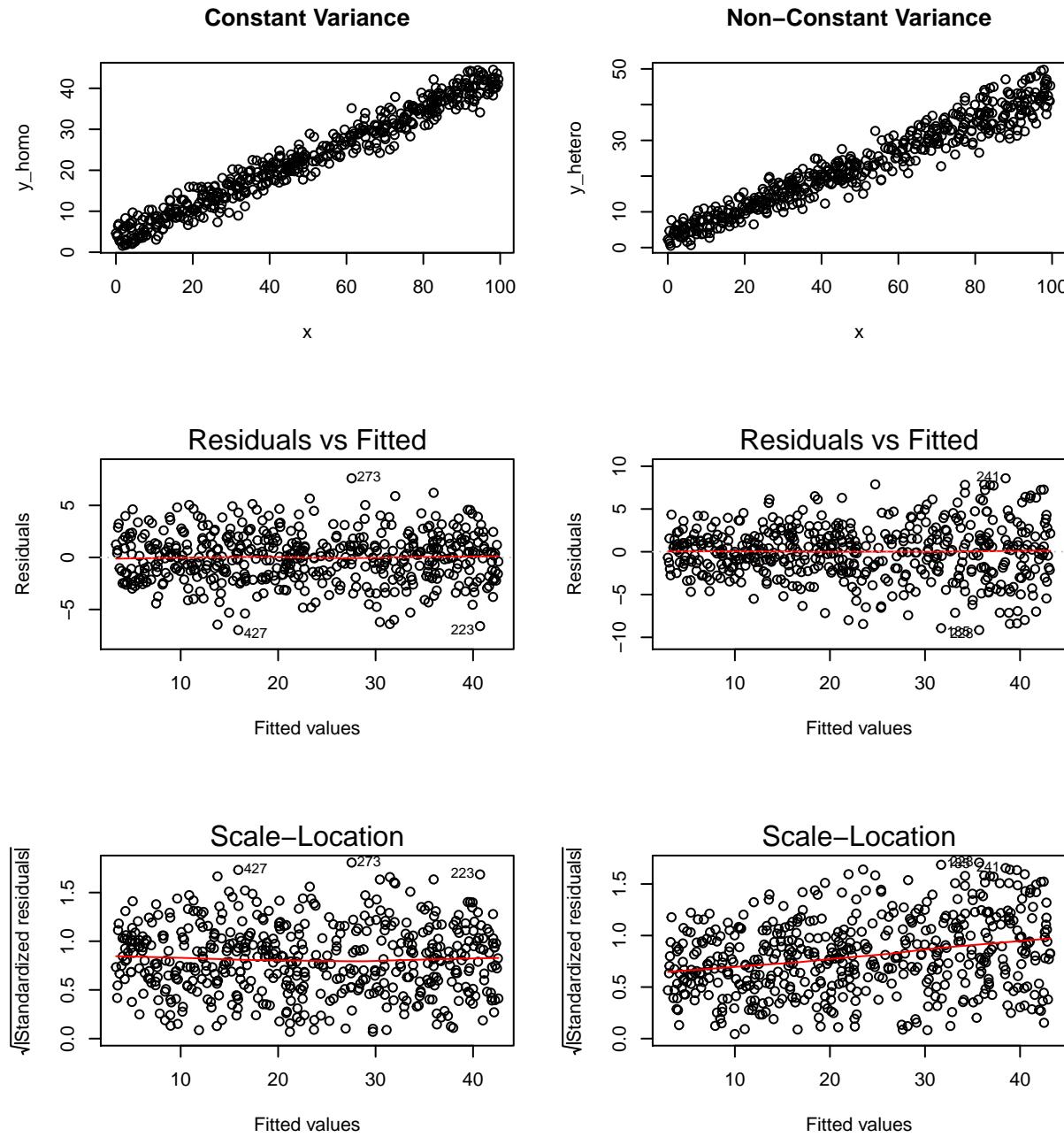
R does provide an additional plot to help assess this issue as linear model diagnostic plot 3. This one looks at the square root of the standardized residual plotted against the fitted values. You want the loess smooth in this plot to be flat, rather than sloped.

```
plot(lm(recov.score ~ dose + age + weight, data = hydrate), which=3)
```



#### 42.4.2 Assessing the Equal Variances Assumption

It's helpful to see how this works in practice. Here are three sets of plots for two settings, where the left plots in each pair show simulated data that are homoscedastic (variance is constant across the levels of the predictor) and the right plots show data that are heteroscedastic (variance is not constant across predictor levels.)



Note the funnel shape for the upper two heteroscedastic plots, and the upward sloping loess line in the last one. *Source:* <http://goo.gl/weMI0U> [from stats.stackexchange.com]

## 42.5 The Normality Assumption

If the plot is straight enough, the data are independent, and the plots don't thicken, you can now move on to the final assumption, that of Normality.

We assume that the errors around the idealized regression model at any specified values of the x-variables follow a Normal model. We need this assumption so that we can use a Student's t-model for inference. As

with other times when we've used Student's t, we'll settle for the residuals satisfying the **Nearly Normal condition**. To assess this, we simply look at a histogram or Normal probability plot of the residuals. Note that the Normality Assumption also becomes less important as the sample size grows.

## 42.6 Outlier Diagnostics: Points with Unusual Residuals

A multiple regression model will always have a point which displays the most unusual residual (that is, the residual furthest from zero in either a positive or negative direction.) As part of our assessment of the normality assumption, we will often try to decide whether the residuals follow a Normal distribution by creating a Q-Q plot of standardized residuals.

### 42.6.1 Standardized Residuals

Standardized residuals are scaled to have mean zero and a constant standard deviation of 1, as a result of dividing the original (raw) residuals by an estimate of the standard deviation that uses all of the data in the data set.

The `rstandard` function, when applied to a linear regression model, will generate the standardized residuals, should we want to build a histogram or other plot of the standardized residuals.

If multiple regression assumptions hold, then the standardized residuals (in addition to following a Normal distribution in general) will also have mean zero and standard deviation 1, with approximately 95% of values between -2 and +2, and approximately 99.74% of values between -3 and +3.

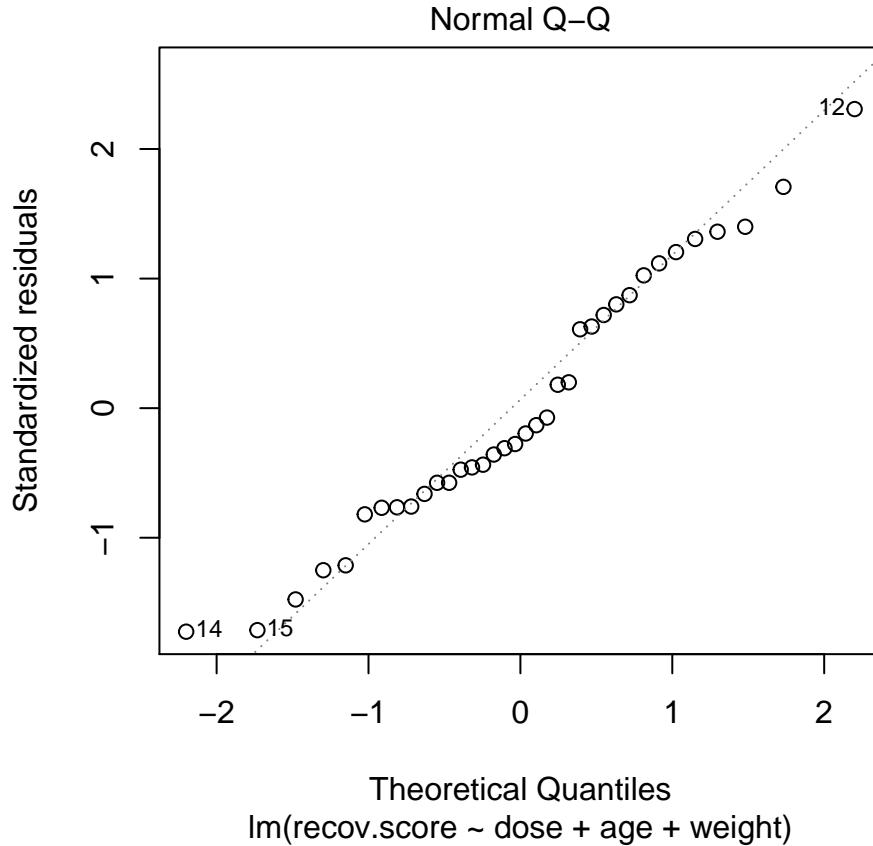
A natural check, therefore, will be to identify the most extreme/unusual standardized residual in our data set, and see whether its value is within the general bounds implied by the Empirical Rule associated with the Normal distribution. If, for instance, we see a standardized residual below -3 or above +3, this is likely to be a very poorly fit point (we would expect to see a point like this about 2.6 times for every 1,000 observations.)

A very poorly fitting point, especially if it also has high influence on the model (a concept we'll discuss shortly), may be sufficiently different from the rest of the points in the data set to merit its removal before modeling. If we did remove such a point, we'd have to have a good reason for this exclusion, and include that explanation in our report.

R's general plot set for a linear model includes (as plot 2) a Normal Q-Q plot of the standardized residuals from the model.

### 42.6.2 Checking the Normality Assumption with a Plot

```
plot(lm(recov.score ~ dose + age + weight, data = hydrate), which=2)
```



In the `hydrate` data, consider our full model with all three predictors. The Q-Q plot of standardized residuals shows the most extreme three cases are in rows 12, 14 and 15. We see from the vertical axis here that none of these points are as large as 3 in absolute value, and only row 12 appears to be above +2 in absolute value.

### 42.6.3 Assessing the Size of the Standardized Residuals

I'll begin by once again placing the details of my linear model into a variable called `modela`. Next, I'll extract, round (to two decimal places) and then sort (from lowest to highest) the standardized residuals from `modela`.

```
modela <- lm(recov.score ~ dose + age + weight, data = hydrate)
sort(round(rstandard(modela), 2))
```

14	15	30	2	19	20	9	10	21	33	11	25
-1.73	-1.71	-1.48	-1.25	-1.21	-0.82	-0.77	-0.77	-0.76	-0.66	-0.58	-0.58
18	7	28	23	13	6	32	16	22	3	5	1
-0.47	-0.46	-0.44	-0.36	-0.31	-0.28	-0.20	-0.13	-0.07	0.18	0.20	0.61
26	36	31	27	34	35	4	24	8	29	17	12
0.63	0.72	0.80	0.87	1.02	1.12	1.20	1.31	1.36	1.40	1.71	2.31

We can see that the smallest residual is -1.73 (in row 14) and the largest is 2.31 (in row 12). Another option would be to look at these rows in terms of the absolute value of their standardized residuals...

```
sort(abs(round(rstandard(modela), 2)))
```

```

22   16    3    5   32    6   13   23   28    7   18   11   25    1   26
0.07 0.13 0.18 0.20 0.20 0.28 0.31 0.36 0.44 0.46 0.47 0.58 0.58 0.61 0.63
 33   36   21    9   10   31   20   27   34   35    4   19    2   24    8
0.66 0.72 0.76 0.77 0.77 0.80 0.82 0.87 1.02 1.12 1.20 1.21 1.25 1.31 1.36
 29   30   15   17   14   12
1.40 1.48 1.71 1.71 1.73 2.31

```

#### 42.6.4 Assessing Standardized Residuals with an Outlier Test

Is a standardized residual of 2.31 particularly unusual in a sample of 36 observations from a Normal distribution, supposedly with mean 0 and standard deviation 1?

No. The `car` library has a test called `outlierTest` which can be applied to a linear model to see if the most unusual studentized residual in absolute value is a surprise given the sample size (the studentized residual is very similar to the standardized residual - it simply uses a different estimate for the standard deviation for every point, in each case excluding the point it is currently studying)

```
outlierTest(lm(recov.score ~ dose + age + weight, data = hydrate))
```

```

No Studentized residuals with Bonferroni p < 0.05
Largest |rstudent|:
  rstudent unadjusted p-value Bonferroni p
12      2.49          0.0184      0.663

```

Our conclusion is that there's no serious problem with normality in this case, and no outliers with studentized (or for that matter, standardized) residuals outside the range we might reasonably expect given a Normal distribution of errors and 36 observations.

## 42.7 Outlier Diagnostics: Identifying Points with Unusually High Leverage

An observation can be an outlier not just in terms of having an unusual residual, but also in terms of having an unusual combination of predictor values. Such an observation is described as having high leverage in a data set.

- R will calculate leverage for the points included in a regression model using the `hatvalues` function as applied to the model.
- The average leverage value is equal to  $k/n$ , where  $n$  is the number of observations included in the regression model, and  $k$  is the number of coefficients (slopes + intercept).
- Any point with a leverage value greater than 2.5 times the average leverage of a point in the data set is one that should be investigated closely.

For instance, in the `hydrate` data, we have 36 observations, so the average leverage will be  $4/36 = 0.111$  and a high leverage point would have leverage  $\geq 10/36$  or .278. We can check this out directly, with a sorted list of leverage values...

```
sort(round(hatvalues(lm(recov.score ~ dose + age + weight, data=hydrate)),3))
```

```

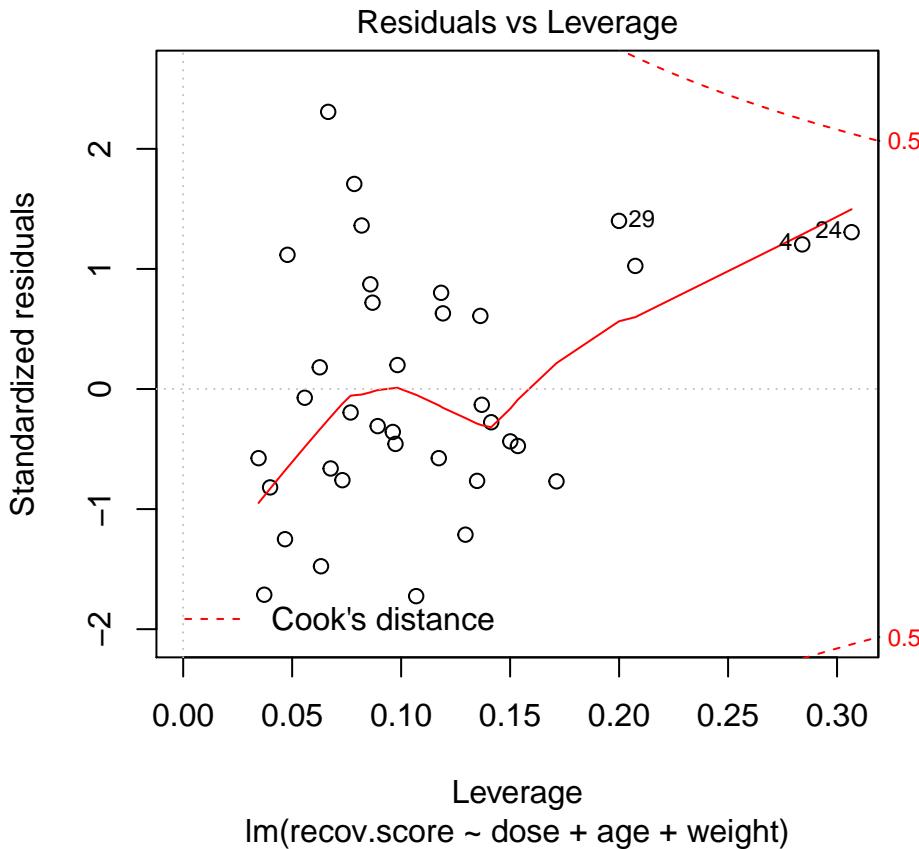
11    15    20     2    35    22     3    30    12    33    21    32
0.035 0.037 0.040 0.047 0.048 0.056 0.063 0.063 0.067 0.068 0.073 0.077
 17     8    27    36    13    23     7     5    14    25    31    26
0.078 0.082 0.086 0.087 0.089 0.096 0.097 0.098 0.107 0.117 0.118 0.119
 19    10     1    16     6    28    18     9    29    34     4    24
0.130 0.135 0.136 0.137 0.141 0.150 0.154 0.171 0.200 0.207 0.284 0.307

```

Two points - 4 and 24 - exceed our cutoff based on having more than 2.5 times the average leverage.

Or we can look at a plot of residuals vs. leverage, which is diagnostic plot 5 for a linear model.

```
plot(lm(recov.score ~ dose + age + weight, data = hydrate), which=5)
```



We see that the most highly leveraged points (those furthest to the right in this plot) are in rows 4 and 24. They are also the two points that meet our 2.5 times the average standard.

To see why points 4 and 24 are the most highly leveraged points, we remember that this just means that these points are unusual in terms of their predictor values.

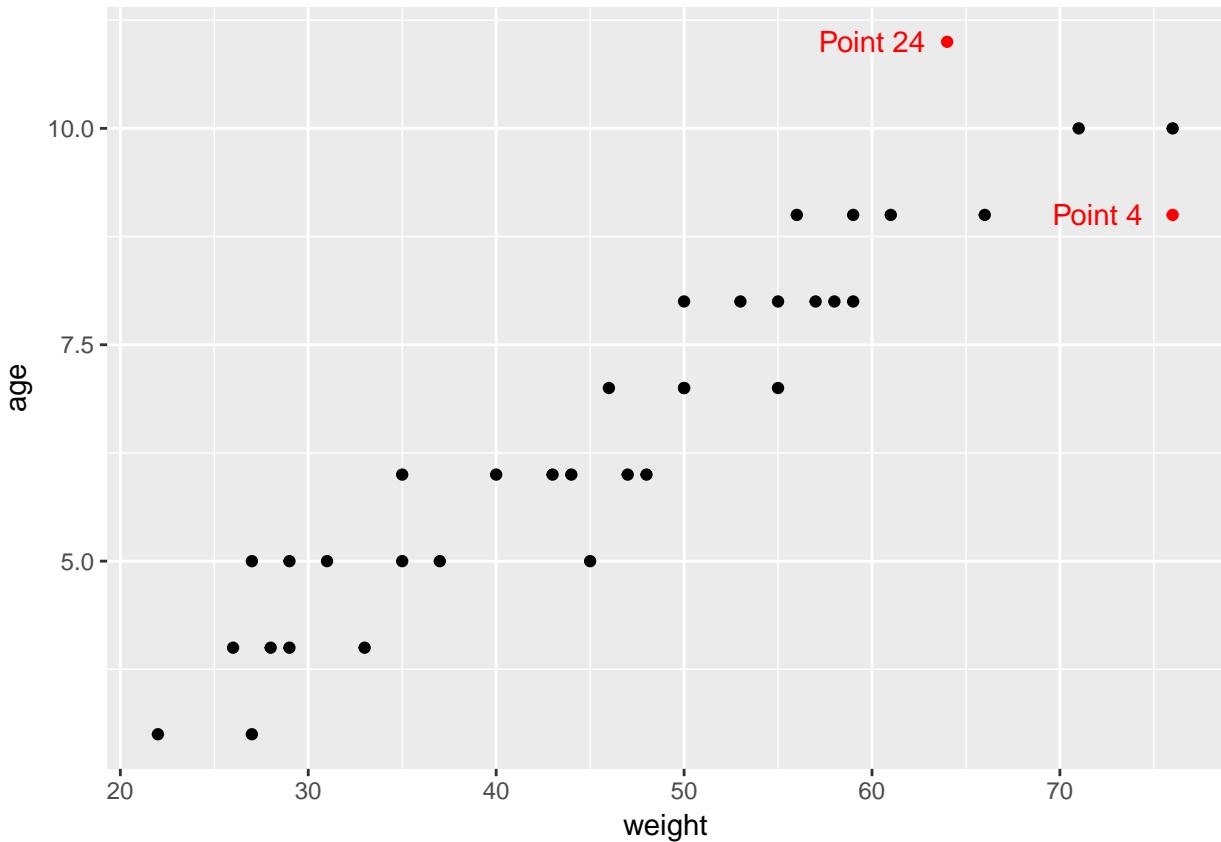
```
hydrate %>%
  filter(id %in% c(4, 24)) %>%
  dplyr::select(id, dose, age, weight)

# A tibble: 2 x 4
  id   dose   age  weight
  <int> <dbl> <int>   <int>
1     4    1.0     9     76
2    24    2.5    11     64

# create indicator for high leverage points
hydrate$hilev <- ifelse(hydrate$id %in% c(4, 24), "Yes", "No")

ggplot(hydrate, aes(x = weight, y = age, color = hilev)) +
```

```
geom_point() +
scale_color_manual(values = c("black", "red")) +
guides(color = FALSE) +
annotate("text", x = 60, y = 11, label = "Point 24", col = "red") +
annotate("text", x = 72, y = 9, label = "Point 4", col = "red")
```



## 42.8 Outlier Diagnostics: Identifying Points with High Influence on the Model

A point in a regression model has high **influence** if the inclusion (or exclusion) of that point will have substantial impact on the model, in terms of the estimated coefficients, and the summaries of fit. The measure I routinely use to describe the level of influence a point has on the model is called **Cook's distance**.

As a general rule, a Cook's distance greater than 1 indicates a point likely to have substantial influence on the model, while a point in the 0.5 to 1.0 range is only occasionally worthy of investigation. Observations with Cook's distance below 0.5 are unlikely to influence the model in any substantial way.

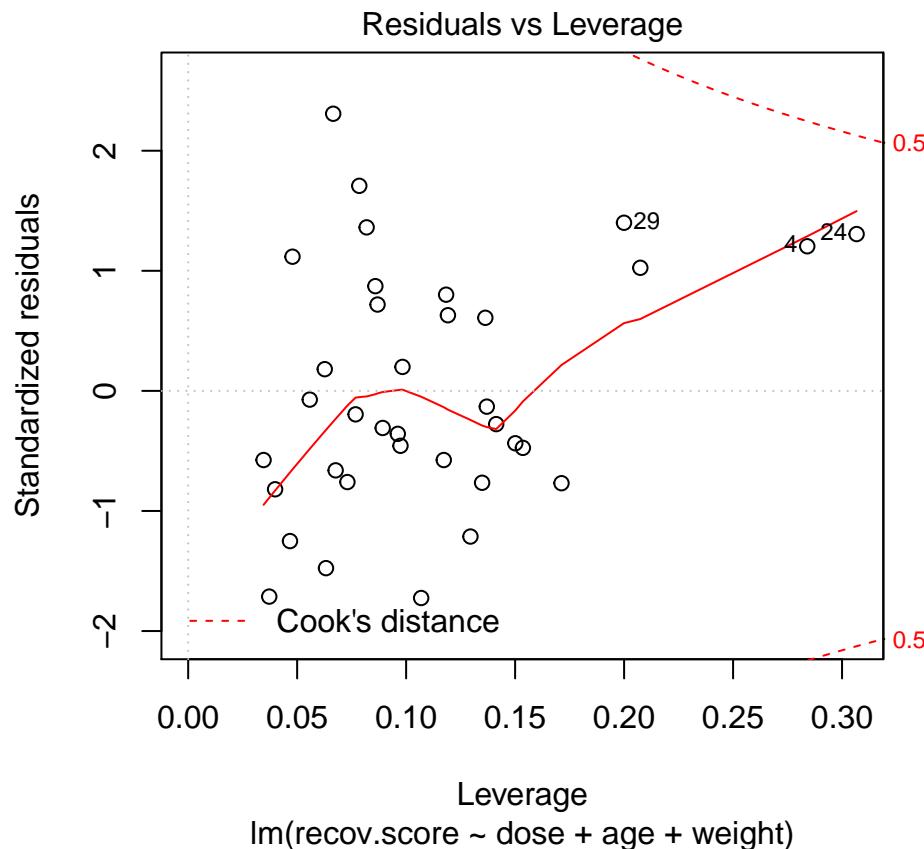
### 42.8.1 Assessing the Value of Cook's Distance

You can obtain information on Cook's distance in several ways in R.

- My favorite is model diagnostic plot 5 (the residuals vs. leverage plot) which uses contours to indicate the value of Cook's distance.

- This is possible because influence, as measured by Cook's distance, is a function of both the observation's standardized residual and its leverage.

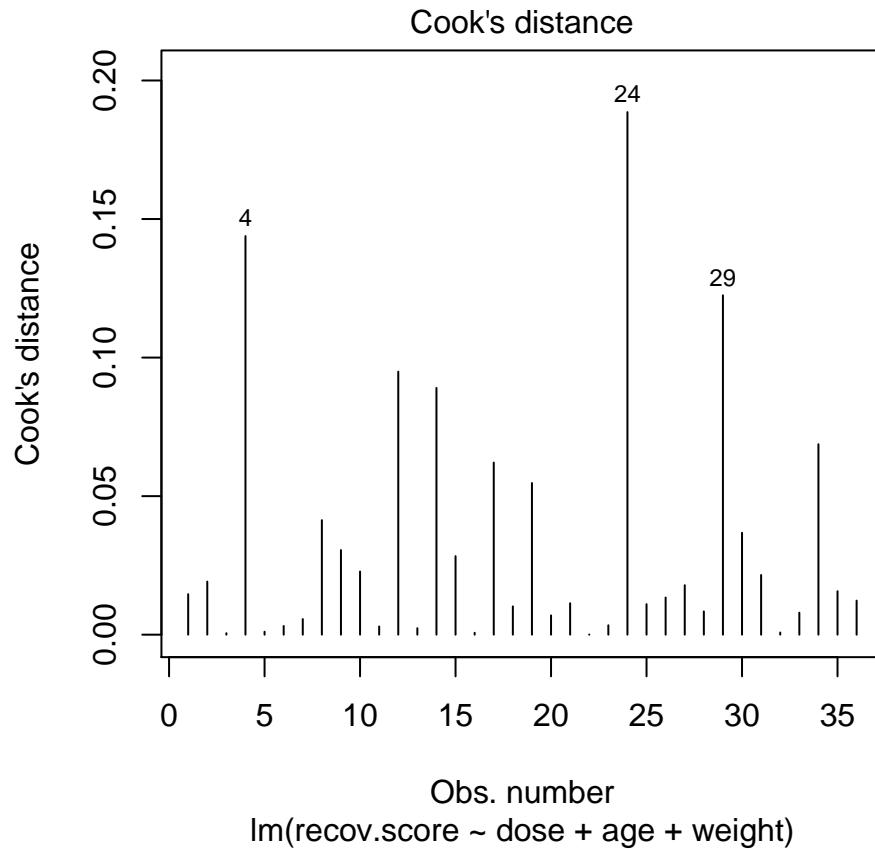
```
plot(lm(recov.score ~ dose + age + weight, data = hydrate), which=5)
```



#### 42.8.2 Index Plot of Cook's Distance

Model Diagnostic Plot 4 for a linear model is an index plot of Cook's distance.

```
plot(lm(recov.score ~ dose + age + weight, data = hydrate), which=4)
```



It is clear from this plot that the largest Cook's distance (somewhere between 0.15 and 0.2) is for the observation in row 24 of the data set.

To see all of the Cook's distances, we can simply ask for them using the `cooks.distance` function.

```
sort(round(cooks.distance(lm(recov.score ~ dose + age + weight), data=hydrate)), 3))
```

22	3	5	16	32	13	6	11	23	7	20	28
0.000	0.001	0.001	0.001	0.001	0.002	0.003	0.003	0.003	0.006	0.007	0.008
33	18	21	25	36	26	1	35	27	2	31	10
0.008	0.010	0.011	0.011	0.012	0.013	0.015	0.016	0.018	0.019	0.022	0.023
15	9	30	8	19	17	34	14	12	29	4	24
0.028	0.031	0.037	0.041	0.055	0.062	0.069	0.089	0.095	0.122	0.144	0.189

## 42.9 Running a Regression Model While Excluding A Point

Suppose that we wanted to remove row 24, the point with the most influence over the model. We could fit a model to the data without row 24 to see the impact...

Note that I have to specify the new data set as `hydrate[-24, ]`: the comma is often forgotten and crucial.

```
summary(lm(recov.score ~ dose + age + weight, data=hydrate[-24,]))
```

```

Call:
lm(formula = recov.score ~ dose + age + weight, data = hydrate[-24,
  ])

Residuals:
    Min      1Q  Median      3Q     Max 
-16.07  -5.61  -2.33   6.94  23.62 

Coefficients:
            Estimate Std. Error t value Pr(>|t|)    
(Intercept)  87.798     6.153   14.27  3.6e-15 ***  
dose         5.824     1.790    3.25   0.0027 **   
age        -1.413     2.596   -0.54   0.5901    
weight       -0.350     0.352   -0.99   0.3279    
---
Signif. codes:  0 '***' 0.001 '**' 0.01 '*' 0.05 '.' 0.1 ' ' 1

Residual standard error: 9.81 on 31 degrees of freedom
Multiple R-squared:  0.481, Adjusted R-squared:  0.431 
F-statistic: 9.57 on 3 and 31 DF,  p-value: 0.000126

```

Compare these results to those obtained with the full data set, shown below.

- How is the model affected by removing point 24?
- What is the impact on the slopes?
- On the summary measures? Residuals?

```
summary(lm(recov.score ~ dose + age + weight, data=hydrate))
```

```

Call:
lm(formula = recov.score ~ dose + age + weight, data = hydrate)

Residuals:
    Min      1Q  Median      3Q     Max 
-16.68  -6.49  -2.20   7.67  22.13 

Coefficients:
            Estimate Std. Error t value Pr(>|t|)    
(Intercept)  85.476     5.965   14.33  1.8e-15 ***  
dose         6.170     1.791    3.45   0.0016 **   
age         0.277     2.285    0.12   0.9043    
weight       -0.543     0.324   -1.68   0.1032    
---
Signif. codes:  0 '***' 0.001 '**' 0.01 '*' 0.05 '.' 0.1 ' ' 1

```

```

Residual standard error: 9.92 on 32 degrees of freedom
Multiple R-squared:  0.458, Adjusted R-squared:  0.408 
F-statistic: 9.03 on 3 and 32 DF,  p-value: 0.000177

```

While it is true that we can sometimes improve the performance of the model in some ways by removing this point, there's no good reason to do so. We can't just remove a point from the data set without a good reason (and, to be clear, "I ran my model and it doesn't fit this point well" is NOT a good reason). Good reasons would include:

- This observation was included in the sample in error, for instance, because the subject was not eligible.

- An error was made in transcribing the value of this observation to the final data set.
- And, sometimes, even “This observation is part of a meaningful subgroup of patients that I had always intended to model separately...” assuming that’s true.

## 42.10 Summarizing Regression Diagnostics for 431

1. Check the “straight enough” condition with scatterplots of the y variable (outcome) against each x-variable (predictor), usually via the top row of a scatterplot matrix.
2. If the data are straight enough (that is, if it looks like the regression model is plausible), fit a regression model, to obtain residuals and influence measures.
  - If not, consider using the Box-Cox approach to identify a possible transformation for the outcome variable, and then recheck the straight enough condition.

The **plot** function for a fitted linear model builds five diagnostic plots.

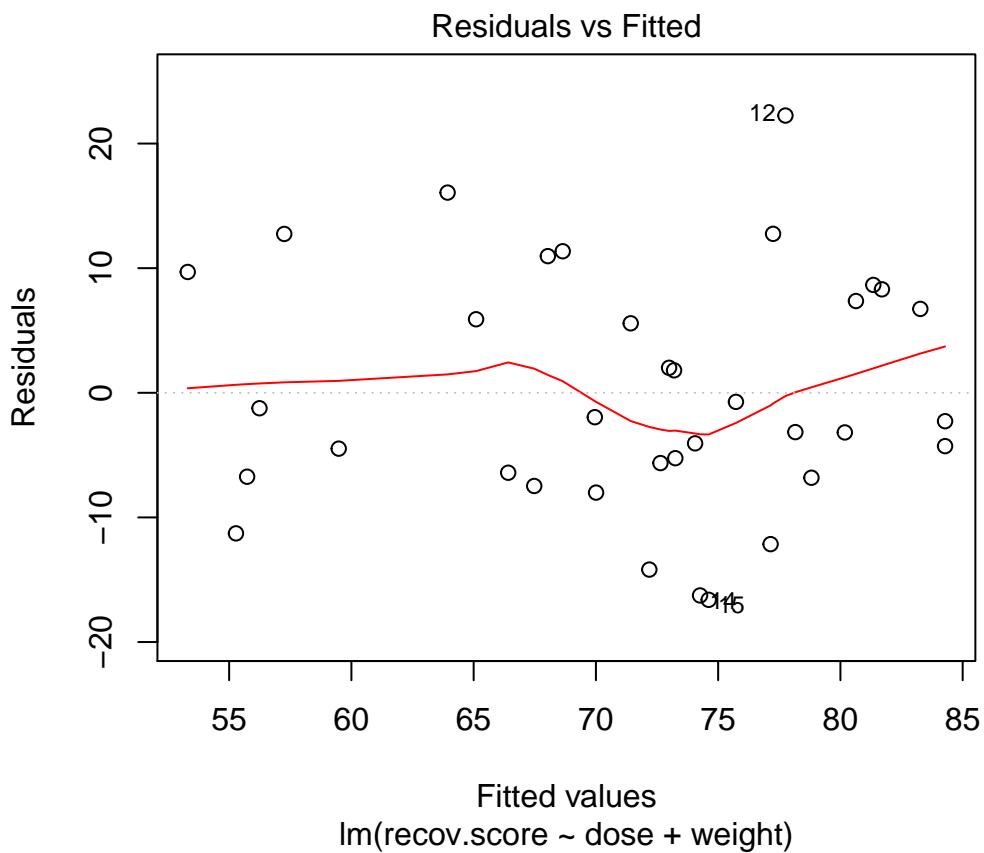
3. [Plot 1] A scatterplot of the residuals against the fitted values.
  - This plot should look patternless. Check in particular for any bend (which would suggest that the data weren’t all that straight after all) and for any thickening, which would indicate non-constant variance.
  - If the data are measured over time, check especially for evidence of patterns that might suggest they are not independent. For example, plot the residuals against time to look for patterns.
4. [Plot 3] A scale-location plot of the square root of the standardized residuals against the fitted values to look for a non-flat loess smooth, which indicates non-constant variance. Standardized residuals are obtained via the `rstandard(model)` function.
5. [Plot 2] If the plots above look OK, then consider a Normal Q-Q plot of the standardized residuals to check the nearly Normal condition.
6. [Plot 5] The final plot we often look at is the plot of residuals vs. leverage, with influence contours. Sometimes, we’ll also look at [Plot 4] the index plot of the Cook’s distance for each observation in the data set.
  - To look for points with substantial **leverage** on the model by virtue of having unusual values of the predictors - look for points whose leverage is at least 2.5 times as large as the average leverage value.
    - The average leverage is always  $k/n$ , where  $k$  is the number of coefficients fit by the model (including the slopes and intercept), and  $n$  is the number of observations in the model.
    - To obtain the leverage values, use the `hatvalues(model)` function.
  - To look for points with substantial **influence** on the model, that is, removing them from the model would change it substantially, consider the Cook’s distance, plotted in contours in Plot 5, or in an index plot in Plot 4.
    - Any Cook’s  $d > 1$  will likely have a substantial impact on the model.
    - Even points with Cook’s  $d > 0.5$  may merit further investigation.
    - Find the Cook’s distances using the `cooks.distance(model)` function.

## 42.11 Back to hydrate: Residual Diagnostics for Dose + Weight Model

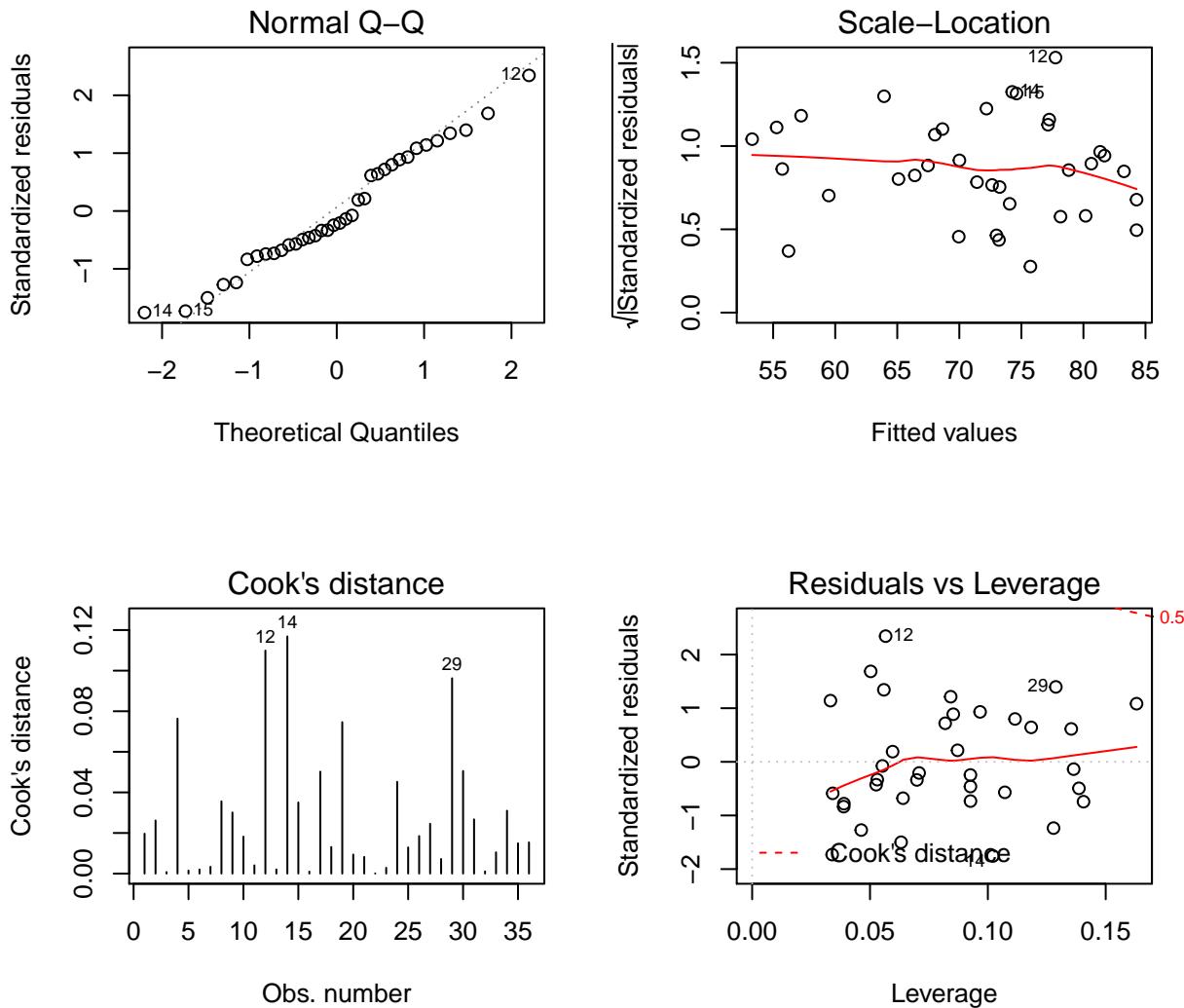
In class, we’ll walk through all five plots produced for the DW model (the model for hydrate recovery score using dose and weight alone).

1. What do these 5 plots tell us about the assumption of linearity?
2. What do these 5 plots tell us about the assumption of independence?
3. What do these 5 plots tell us about the assumption of constant variance?
4. What do these 5 plots tell us about the assumption of Normality?
5. What do these 5 plots tell us about leverage of individual observations?
6. What do these 5 plots tell us about influence of individual observations?

```
plot(lm(recov.score ~ dose + weight, data = hydrate), which=1)
```



```
par(mfrow=c(2,2))
plot(lm(recov.score ~ dose + weight, data = hydrate), which=2:5)
```



## 42.12 Violated Assumptions: Problematic Residual Plots?

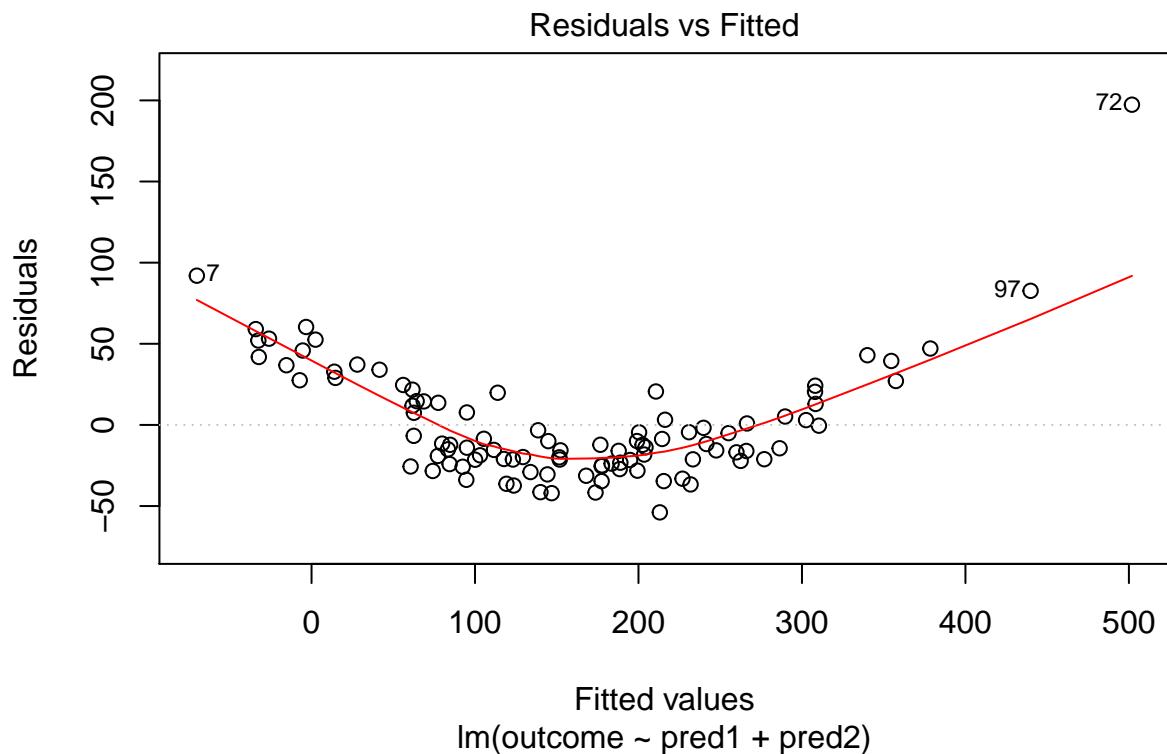
So what do serious assumption violations look like, and what can we do about them?

## 42.13 Problems with Linearity

Here is a simulated example that shows a clear problem with non-linearity.

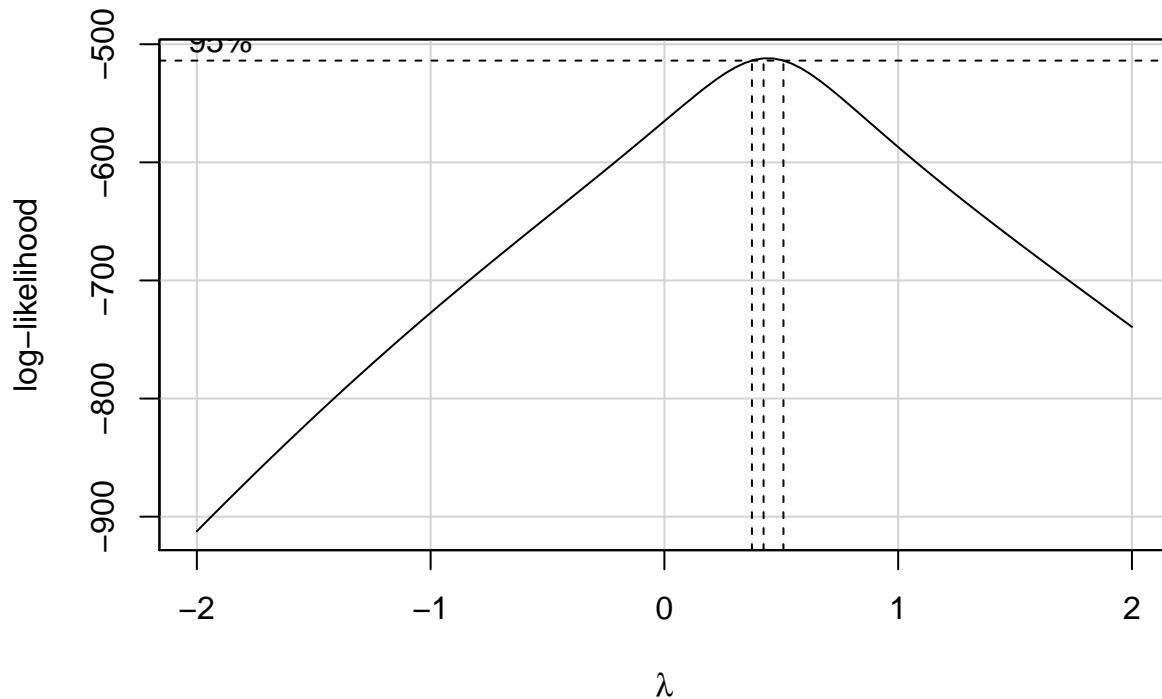
```
set.seed(4311); x1 <- rnorm(n = 100, mean = 15, sd = 5)
set.seed(4312); x2 <- rnorm(n = 100, mean = 10, sd = 5)
set.seed(4313); e1 <- rnorm(n = 100, mean = 0, sd = 15)
y <- 15 + x1 + x2^2 + e1
viol1 <- data.frame(outcome = y, pred1 = x1, pred2 = x2) %>% tbl_df
```

```
model.1 <- lm(outcome ~ pred1 + pred2, data = viol1)
plot(model.1, which = 1)
```



In light of this, I would be looking for a potential transformation of outcome. Does the Box-Cox plot make any useful suggestions?

```
boxCox(model.1); powerTransform(model.1)
```

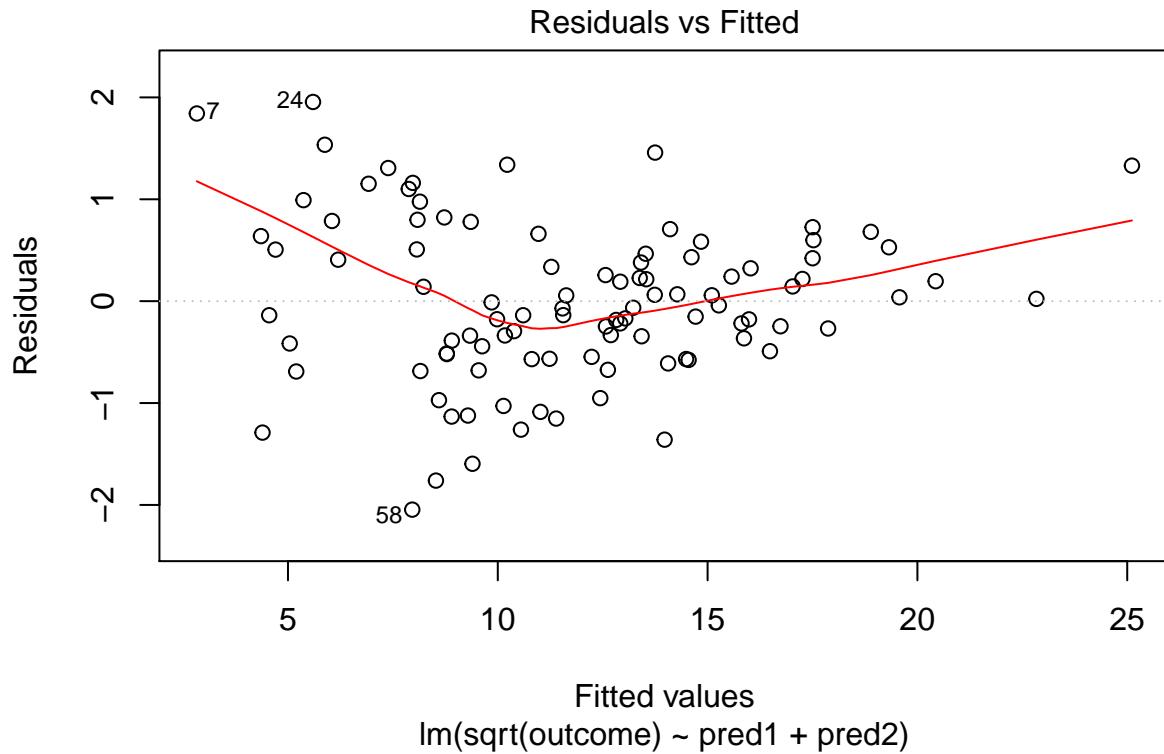


Estimated transformation parameters

```
Y1  
0.441
```

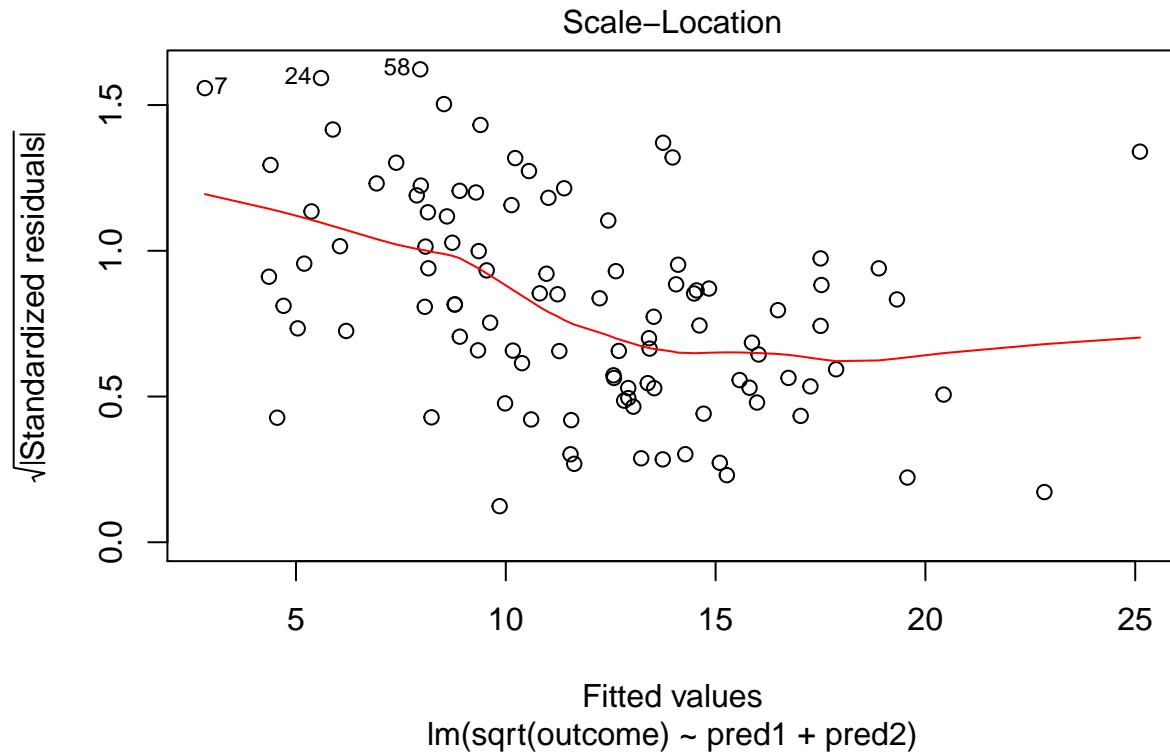
Note that if the outcome was negative, we would have to add some constant value to every outcome in order to get every outcome value to be positive, and Box-Cox to run. This suggests fitting a new model, using the square root of the outcome.

```
model.2 <- lm(sqrt(outcome) ~ pred1 + pred2, data = viol1)  
plot(model.2, which = 1)
```



This is meaningfully better in terms of curve, but now looks a bit fan-shaped, indicating a potential problem with heteroscedasticity. Let's look at the scale-location plot for this model.

```
plot(model.2, which = 3)
```

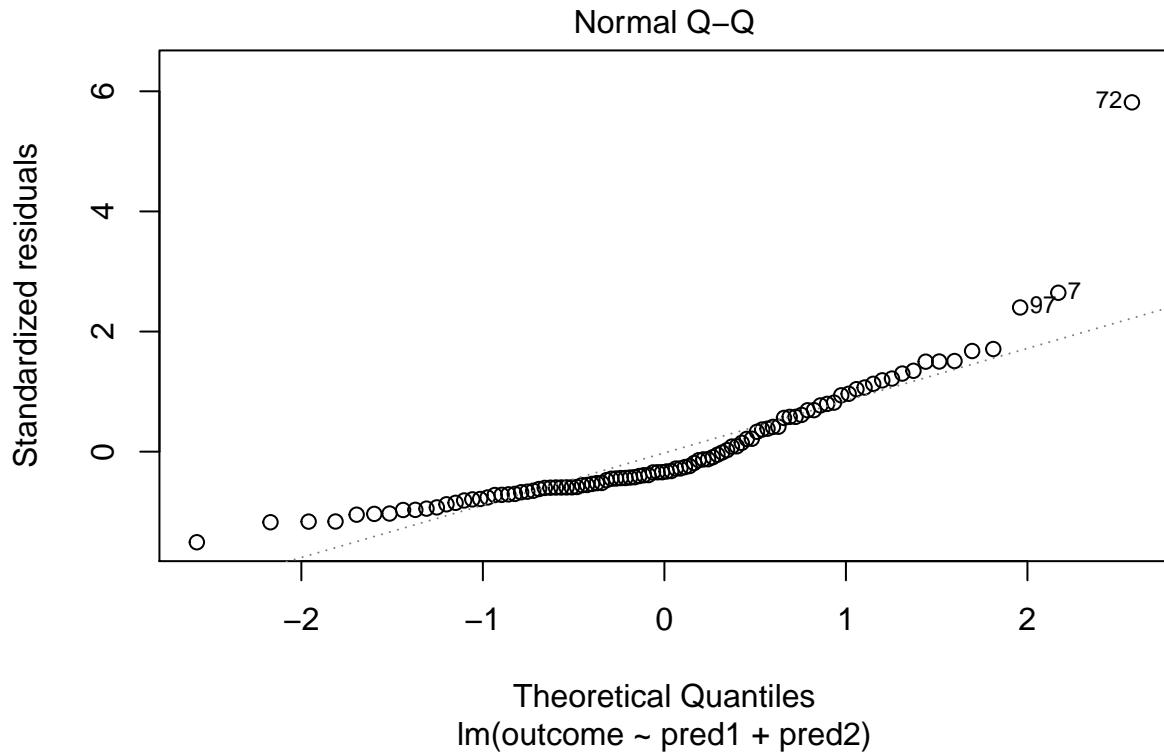


This definitely looks like there's a trend down in this plot. So the square root transformation, by itself, probably hasn't resolved assumptions sufficiently well. We'll have to be very careful about our interpretation of the model.

## 42.14 Problems with Non-Normality: An Influential Point

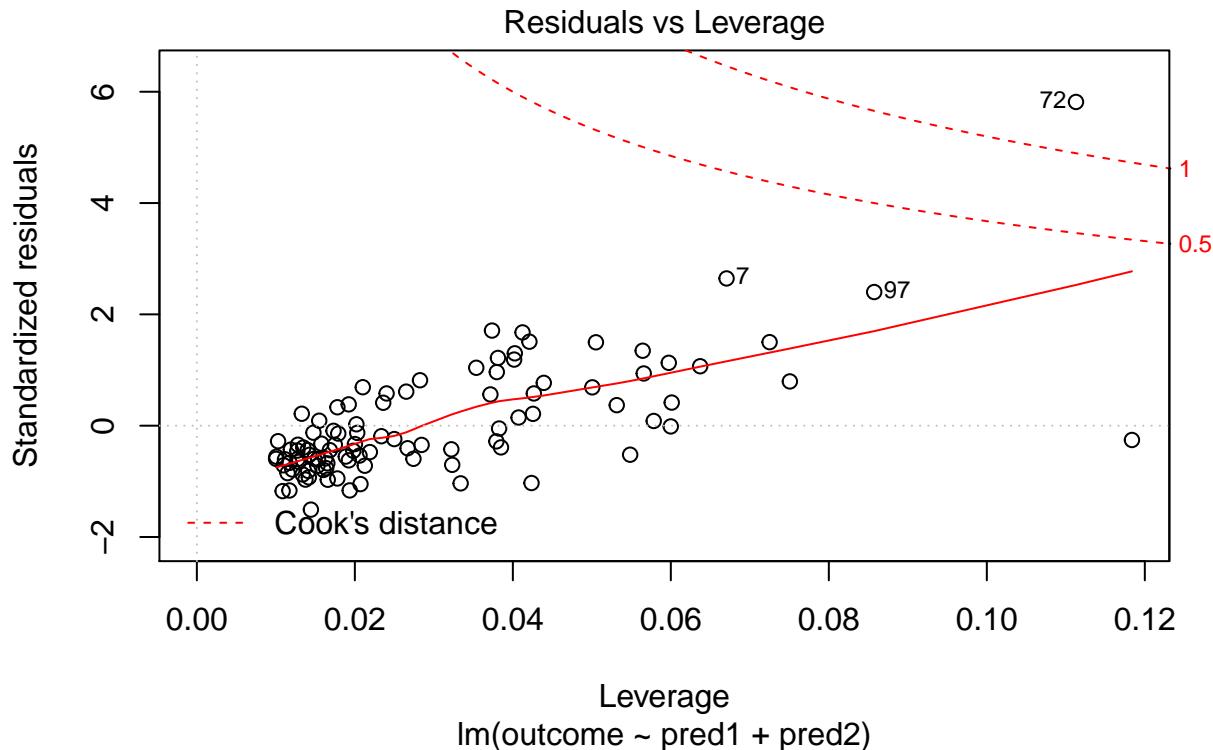
With 100 observations, a single value with a standardized residual above 3 is very surprising. In our initial `model.1` here, we have a standardized residual value as large as 6, so we clearly have a problem with that outlier.

```
plot(model.1, which = 2)
```



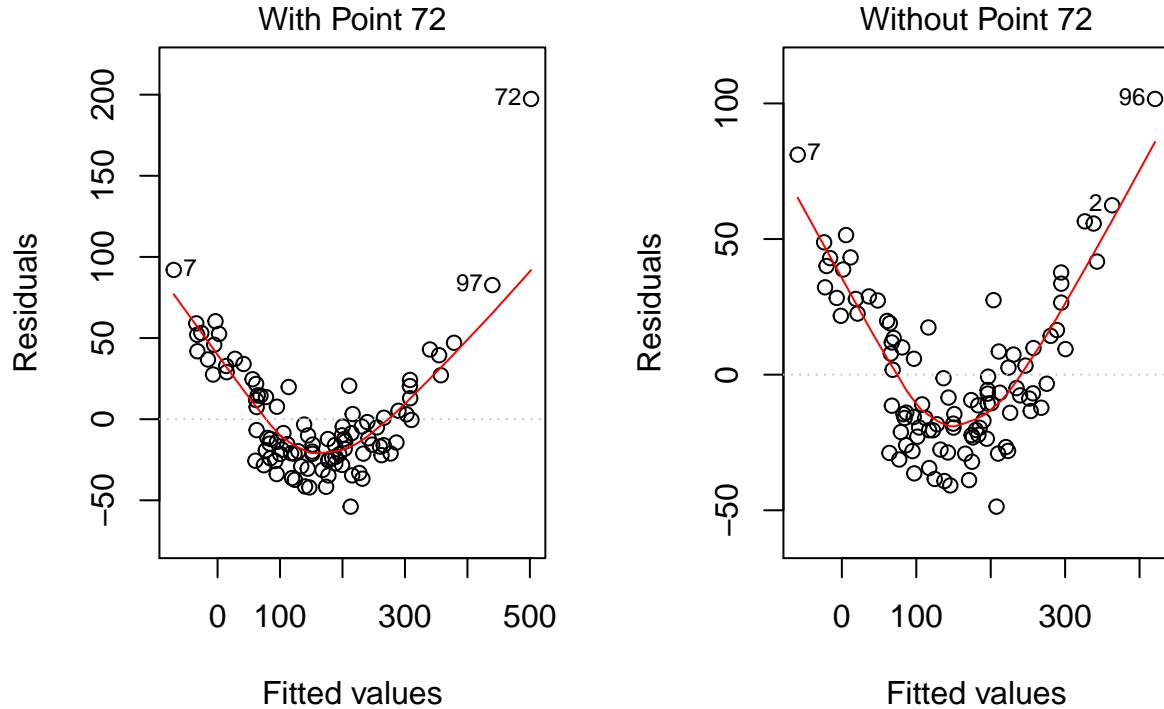
Should we, perhaps, remove point 72, and try again? Only if we have a reason beyond “it was poorly fit” to drop that point. Is point 72 highly leveraged or influential?

```
plot(model.1, which = 5)
```



What if we drop this point (72) and fit our linear model again. Does this resolve our difficulty with the assumption of linearity?

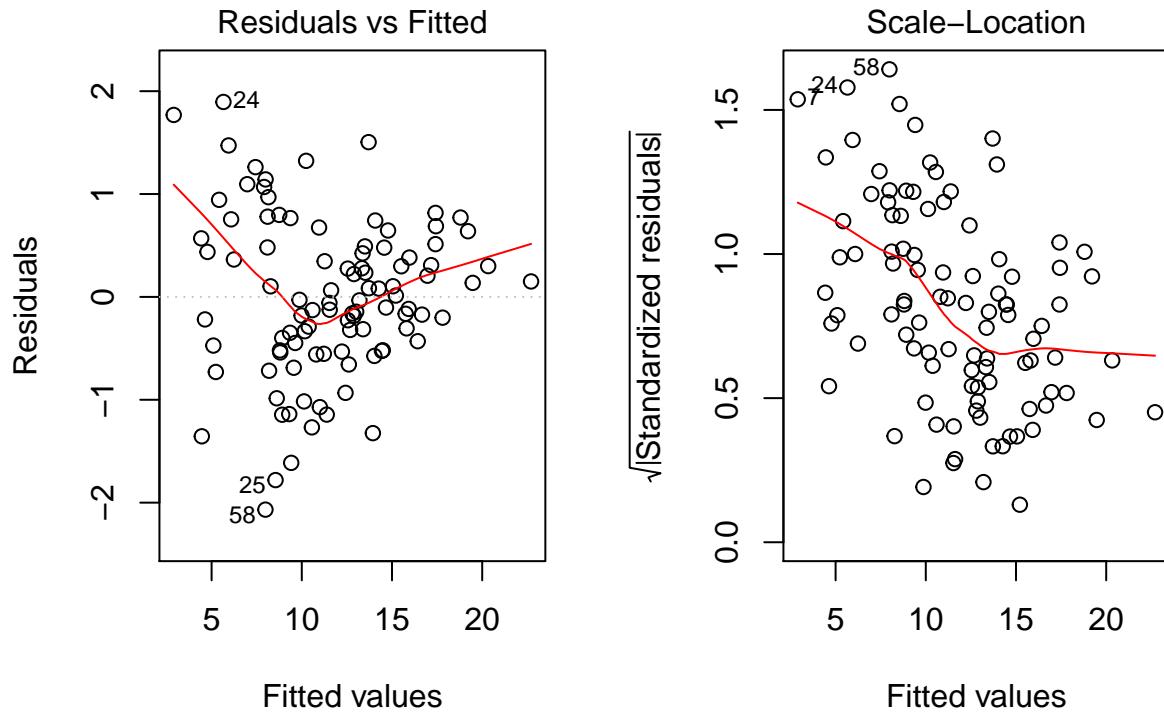
```
model.1.no72 <- lm(outcome ~ pred1 + pred2, data = viol1[-72,])
par(mfrow=c(1,2))
plot(model.1, which = 1, caption = "With Point 72")
plot(model.1.no72, which = 1, caption = "Without Point 72")
```



```
par(mfrow=c(1,1))
```

No, it doesn't. But what if we combine our outcome transformation with dropping point 72?

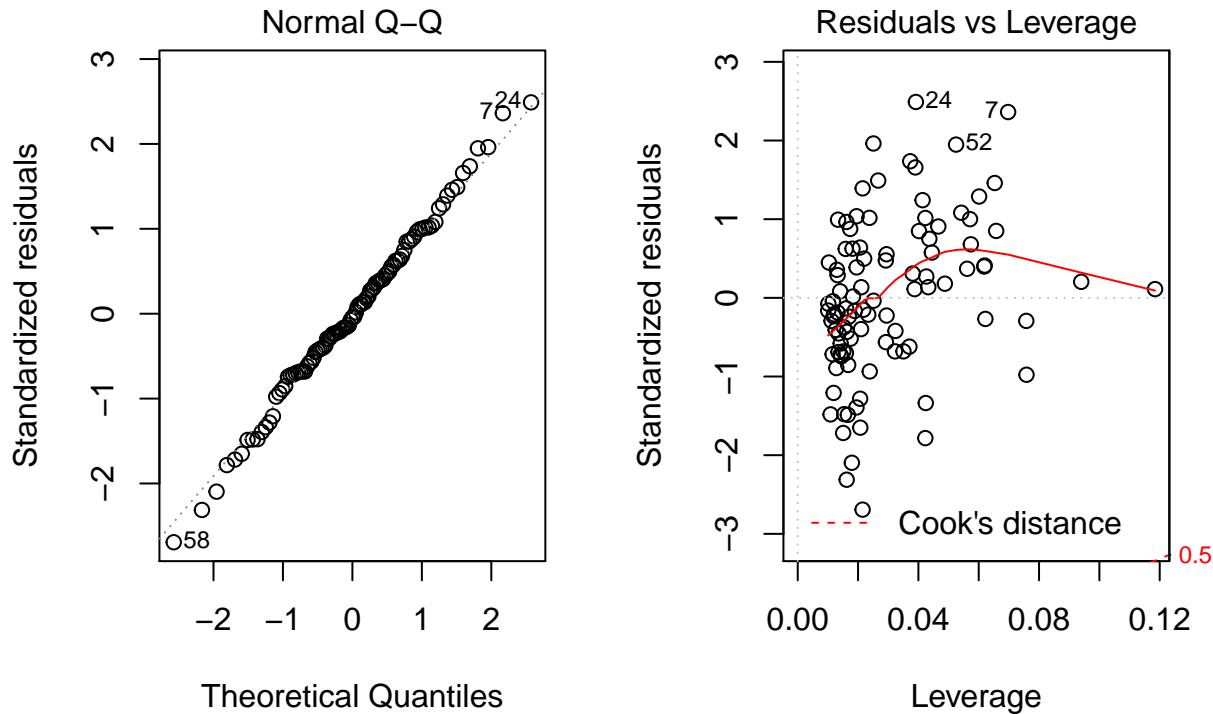
```
model.2.no72 <- lm(sqrt(outcome) ~ pred1 + pred2, data = viol1[-72,])
par(mfrow=c(1,2))
plot(model.2.no72, which = c(1,3))
```



```
par(mfrow=c(1,1))
```

Nope. That still doesn't alleviate the problem of heteroscedasticity very well. At least, we no longer have any especially influential points, nor do we have substantial non-Normality.

```
par(mfrow=c(1,2))
plot(model.2.no72, which = c(2,5))
```

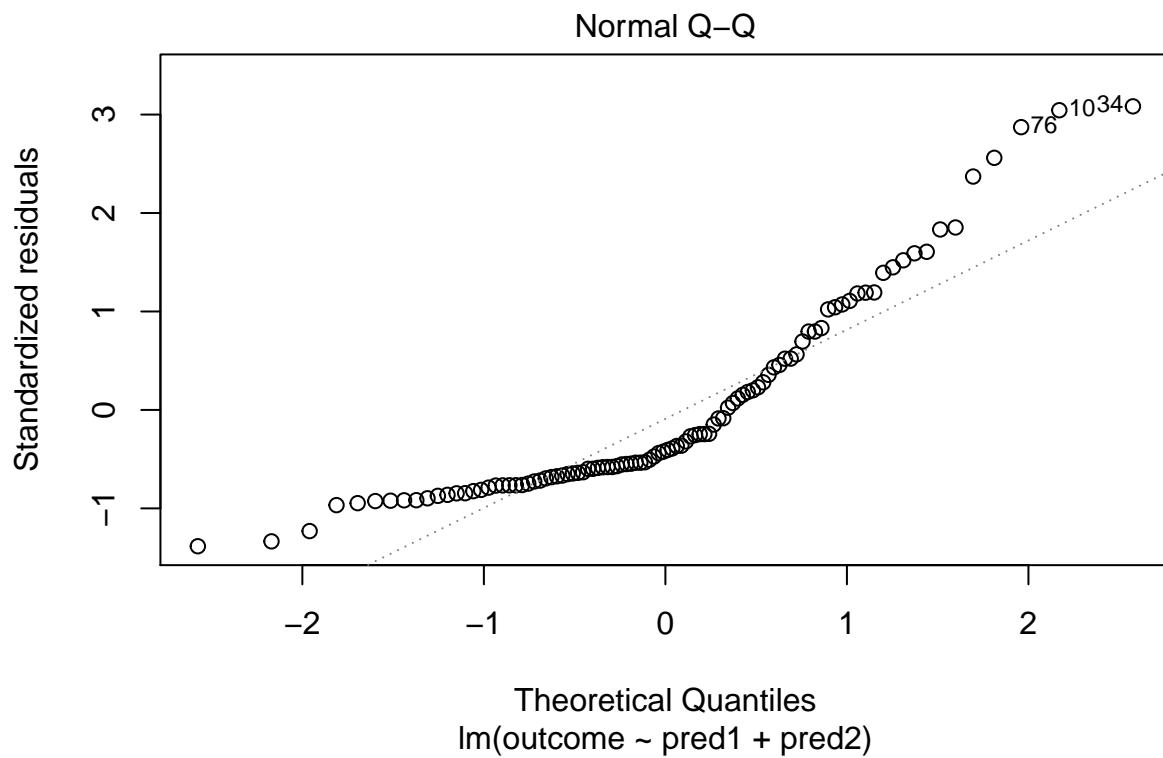


```
par(mfrow=c(1,1))
```

At this point, I would be considering potential transformations of the predictors, quite possibly fitting some sort of polynomial term or cubic spline term in the predictors, but I'll leave that for discussion in 432.

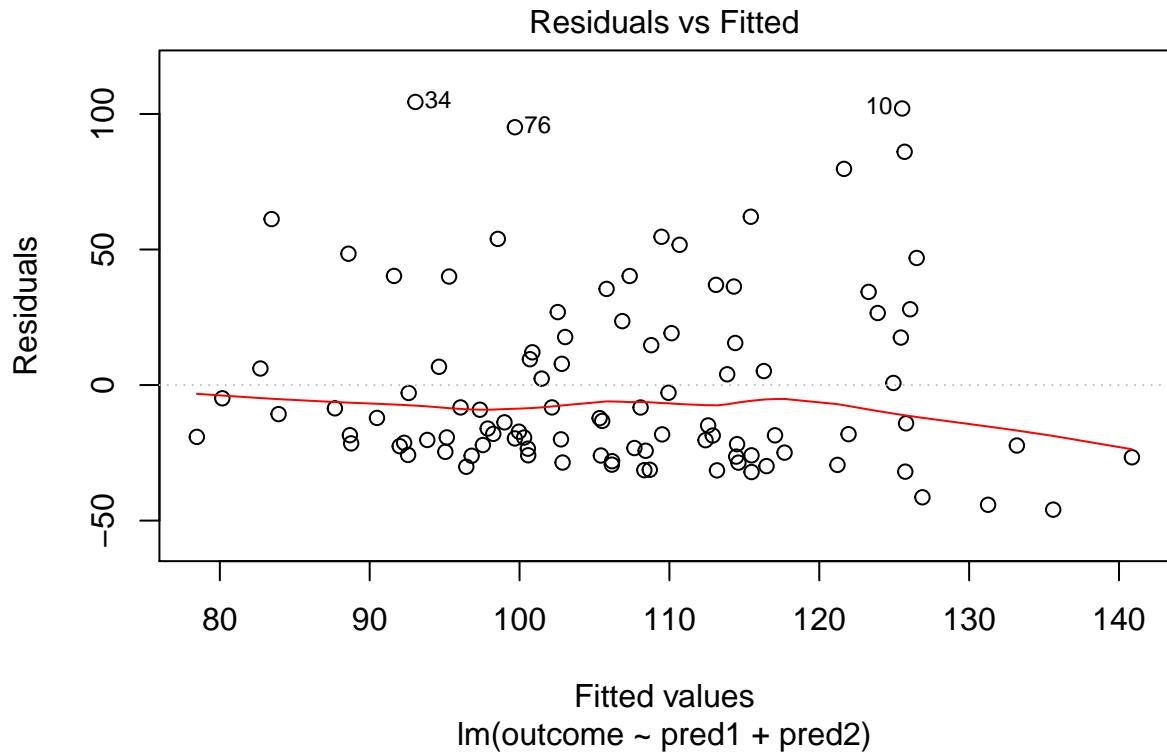
## 42.15 Problems with Non-Normality: Skew

```
set.seed(4314); x1 <- rnorm(n = 100, mean = 15, sd = 5)
set.seed(4315); x2 <- rnorm(n = 100, mean = 10, sd = 5)
set.seed(4316); e2 <- rnorm(n = 100, mean = 3, sd = 5)
y2 <- 50 + x1 + x2 + e2^2
viol2 <- data.frame(outcome = y2, pred1 = x1, pred2 = x2) %>% tbl_df
model.3 <- lm(outcome ~ pred1 + pred2, data = viol2)
plot(model.3, which = 2)
```



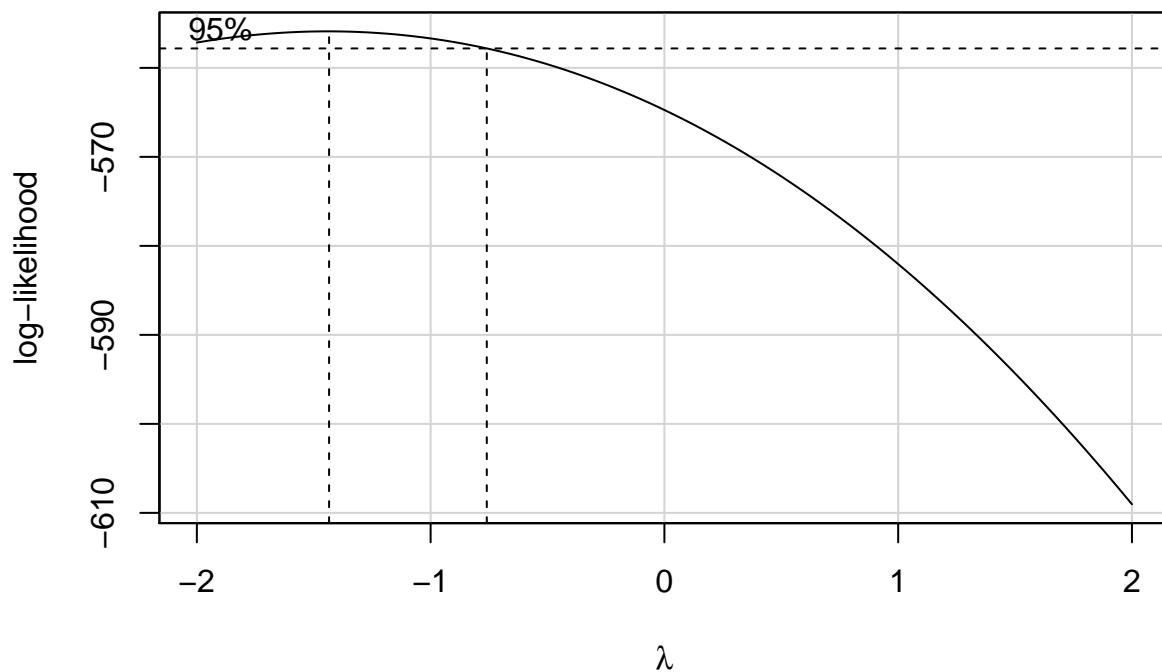
Skewed residuals often show up in strange patterns in the plot of residuals vs. fitted values, too, as in this case.

```
plot(model.3, which = 1)
```



Clearly, we have some larger residuals on the positive side, but not on the negative side. Would an outcome transformation be suggested by Box-Cox?

```
boxCox(model.3); powerTransform(model.3)
```

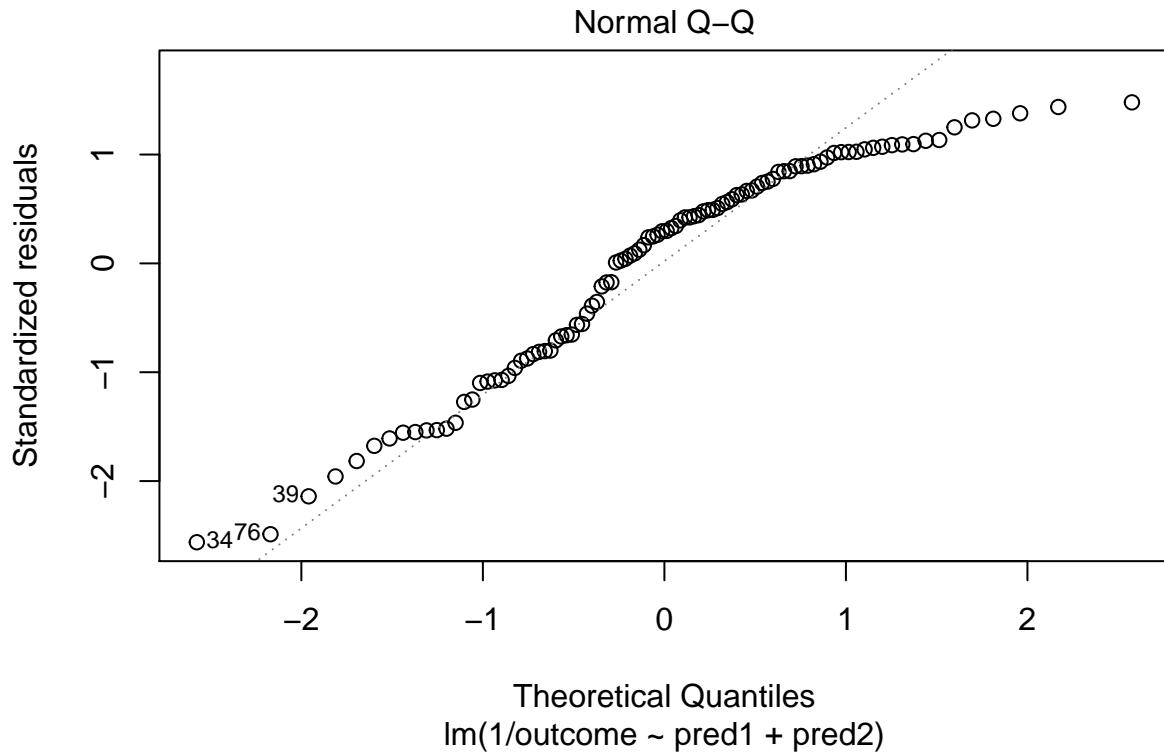


Estimated transformation parameters

```
Y1  
-1.44
```

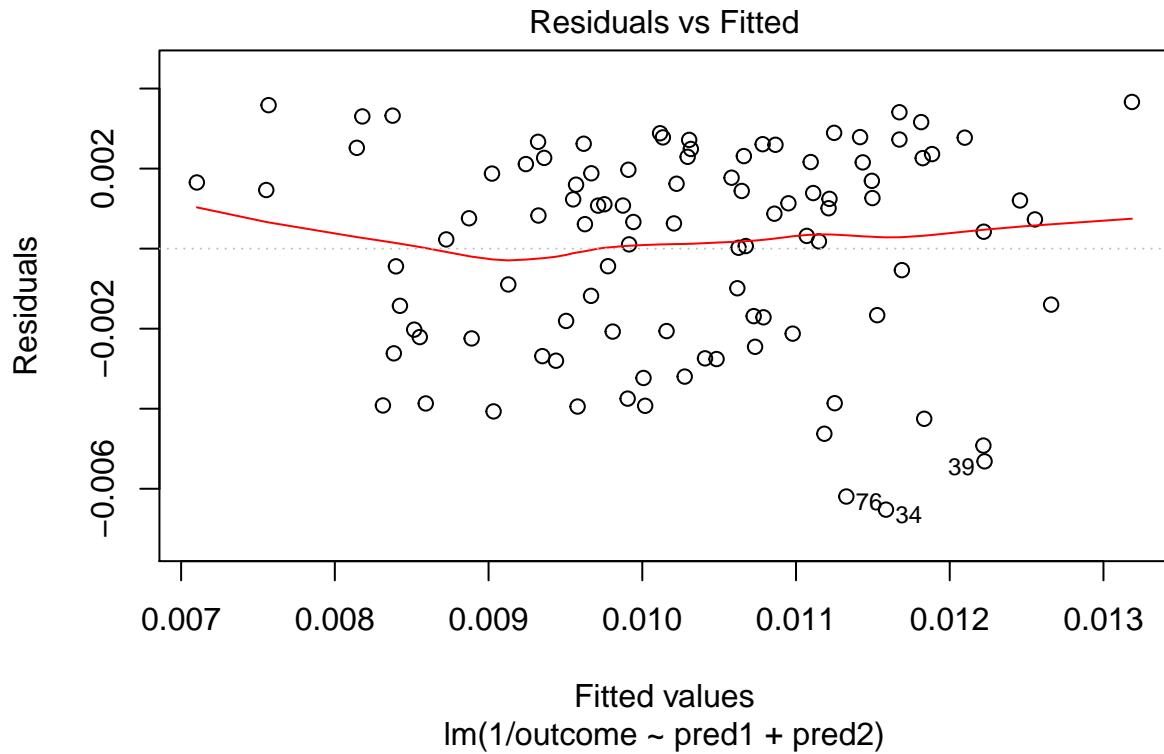
The suggested transformation is either the inverse or the inverse square of our outcome. Let's try the inverse.

```
model.4 <- lm(1/outcome ~ pred1 + pred2, data = viol2)  
plot(model.4, which = 2)
```



OK. That's something of an improvement. How about the other residual plots with this transformation?

```
plot(model.4, which = 1)
```



The impact of the skew is reduced, at least. I might well be satisfied enough with this, in practice.



## Chapter 43

# Model Selection and Out-of-Sample Validation

Sometimes, we use a regression model for description of a multivariate relationship which requires only that we provide an adequate fit to the data at hand. Should we want to use the model for prediction, then a more appropriate standard which requires us to fit a model that does each of the following three things:

1. [fits well] Provides an adequate fit to the data at hand
2. [parsimonious] Includes only those predictors which actually have detectable predictive value
3. [predicts well out of sample] Does a better job predicting our outcome in new data than other alternative models

We'll spend considerable time in 432 studying how best to validate a regression model, but for now, we'll focus on a pair of issues:

- a. Given a set of predictors, how should we let the computer help us understand which subsets of those predictors are most worthy of our additional attention?
- b. Given a pair of models to compare, what tools should we use to determine which one better predicts new data?

### 43.1 Using the WCGS Data to predict Cholesterol Level

To address these issues, I'll look at one of our old examples: the `wcgs` data (Western Collaborative Group Study), described in Section 13. We'll try to predict the variable `chol` on the basis of some subset of the following five predictors: `age`, `bmi`, `sbp`, `dbp` and `smoke`.

The steps are:

0. Check the `wcgs` data for missing or out-of-range values in the variables under study<sup>1</sup>.
1. Separate the `wcgs` data into a test sample of 500 observations selected at random, and a model development (training) sample consisting of the remaining 2654 observations.
2. Using only the model development sample, run a stepwise regression algorithm working off of the kitchen sink model using backwards selection to identify a reasonable candidate for a model. Call this model A.
3. Develop a second potential model (called model B) for the model development data by eliminating the least clearly helpful predictor in model A.
4. Use the AIC to compare model A to model B in the development sample.

---

<sup>1</sup>Actually, I will skip this range and missingness check here, and will wind up regretting that later.

5. Finally, moving forward to the holdout sample, compare the quality of predictions made by model A to those made by model B in terms of two of the many possible criteria:

- [i] mean squared prediction error and
- [ii] mean absolute prediction error
- to see if either model (A or B) is clearly superior in terms of out-of-sample prediction.

## 43.2 Separating the Data into a Training and a Test Sample

There are several ways to partition the data into training (model development) and test (model checking) samples. For example, we could develop separate data frames for a holdout sample of 500 randomly selected subjects (`wcgs.test`), and then use the remainder as the model development sample (`wcgs.dev`). Remember to set a seed so that you can replicate the selection.

```
set.seed(431); wcgs.test <- wcgs %>% sample_n(500)
## hold out exactly 500 randomly selected observations

wcgs.dev <- anti_join(wcgs, wcgs.test, "id")
## model development sample - 2654 observations

wcgs.test

# A tibble: 500 x 22
   id    age   agec height weight lnwght wghtcat   bmi    sbp lnsbp    dbp
   <int> <int> <fctr> <int> <int>   <dbl> <fctr> <dbl> <int> <dbl> <int>
 1 10191    50 46-50     71    190    5.25 170-200  26.5    120   4.79    80
 2 13237    44 41-45     70    168    5.12 140-170  24.1    138   4.93    74
 3 13361    52 51-55     70    176    5.17 170-200  25.3    110   4.70    78
 4 10265    43 41-45     72    147    4.99 140-170  19.9    124   4.82    80
 5 10207    46 46-50     64    136    4.91 < 140   23.3    122   4.80    80
 6 13463    55 51-55     66    165    5.11 140-170  26.6    144   4.97    94
 7 21063    45 41-45     71    165    5.11 140-170  23.0    136   4.91    82
 8 12260    45 41-45     69    145    4.98 140-170  21.4    118   4.77    84
 9 10025    43 41-45     69    190    5.25 170-200  28.1    138   4.93    90
10 6047     50 46-50     72    190    5.25 170-200  25.8    136   4.91    92
# ... with 490 more rows, and 11 more variables: chol <int>,
#   behpat <fctr>, dibpat <fctr>, smoke <fctr>, ncigs <int>, arcus <int>,
#   chd69 <fctr>, typchd69 <int>, time169 <int>, t1 <dbl>, uni <dbl>
dim(wcgs.dev) # verify size of development sample
```

[1] 2654 22

### 43.2.1 Using a specified fraction of the data in the test sample

If we'd wanted to select 20% of the data for our test sample, we could have instead used the `sample_frac` and `anti_join` commands. For the `wcgs` data which has a unique `id` variable that identifies each subject, we'd have...

```
set.seed(43199); wcgs.train80 <- wcgs %>% sample_frac(0.80)
wcgs.test20 <- anti_join(wcgs, wcgs.train80, by="id")
dim(wcgs.train80)
```

[1] 2523 22

```
dim(wcgs.test20)
```

```
[1] 631 22
```

Given a large sample size (at least 500 observations in the full data set) I would usually think about holding out somewhere between 15% and 25% of the data in this manner.

### 43.3 Stepwise Regression to Select Predictors

We next select the `wcgs.dev` (development sample) and run a stepwise procedure, beginning with the kitchen sink model, that includes all potential predictors.

#### 43.3.1 The Kitchen Sink Model

```
summary(lm(chol ~ age + bmi + sbp + dbp + smoke, data = wcgs.dev))
```

Call:

```
lm(formula = chol ~ age + bmi + sbp + dbp + smoke, data = wcgs.dev)
```

Residuals:

Min	1Q	Median	3Q	Max
-130.96	-28.13	-2.33	25.90	173.24

Coefficients:

	Estimate	Std. Error	t value	Pr(> t )
(Intercept)	138.4118	11.1947	12.36	< 2e-16 ***
age	0.4788	0.1497	3.20	0.00139 **
bmi	0.5222	0.3418	1.53	0.12675
sbp	0.0539	0.0856	0.63	0.52918
dbp	0.5031	0.1353	3.72	0.00021 ***
smokeYes	10.2623	1.6693	6.15	9.1e-10 ***

---

Signif. codes: 0 '\*\*\*' 0.001 '\*\*' 0.01 '\*' 0.05 '.' 0.1 ' ' 1

Residual standard error: 42.1 on 2638 degrees of freedom

(10 observations deleted due to missingness)

Multiple R-squared: 0.0379, Adjusted R-squared: 0.0361

F-statistic: 20.8 on 5 and 2638 DF, p-value: <2e-16

#### 43.3.2 Stepwise (Backward Elimination) Procedure

```
step(lm(chol ~ age + bmi + sbp + dbp + smoke, data = wcgs.dev))
```

Start: AIC=19784

chol ~ age + bmi + sbp + dbp + smoke

	Df	Sum of Sq	RSS	AIC
- sbp	1	702	4677574	19782
<none>		4676872	19784	

```
- bmi     1      4137 4681009 19784
- age     1      18148 4695021 19792
- dbp     1      24507 4701379 19796
- smoke   1      67002 4743874 19820
```

Step: AIC=19782  
chol ~ age + bmi + dbp + smoke

	Df	Sum of Sq	RSS	AIC
<none>		4677574	19782	
- bmi	1	4370	4681944	19783
- age	1	18964	4696538	19791
- dbp	1	69038	4746612	19819
- smoke	1	69338	4746912	19819

Call:  
`lm(formula = chol ~ age + bmi + dbp + smoke, data = wcgs.dev)`

Coefficients:

(Intercept)	age	bmi	dbp	smokeYes
139.377	0.487	0.536	0.566	10.377

The stepwise process first eliminates `sbp` from the model, then sees no substantial improvement in AIC after this has been done, so it lands on a four-predictor model with `age`, `bmi`, `dbp` and `smoke`.

### 43.3.3 Three Candidate Models

For purposes of this exercise, we'll call this four-predictor model `model.A` and compare it to a three-predictor model with `age`, `dbp` and `smoke`, which we'll call `model.B`

```
model.kitchensink <- lm(chol ~ age + bmi + sbp + dbp + smoke, data = wcgs.dev)
model.A <- lm(chol ~ age + bmi + dbp + smoke, data = wcgs.dev)
model.B <- lm(chol ~ age + dbp + smoke, data = wcgs.dev)
```

## 43.4 AIC, ANOVA and BIC to assess Candidate Models

The stepwise regression output specifies the AIC value for each model, but we can also look at other characteristics, like the ANOVA table comparing the various models, or the Bayesian Information Criterion, abbreviated BIC.

```
AIC(model.kitchensink, model.A, model.B)
```

	df	AIC
model.kitchensink	7	27289
model.A	6	27288
model.B	5	27288

AIC suggests model A (since it has the smallest AIC of these choices)

```
anova(model.kitchensink, model.A, model.B)
```

Analysis of Variance Table

```
Model 1: chol ~ age + bmi + sbp + dbp + smoke
```

```

Model 2: chol ~ age + bmi + dbp + smoke
Model 3: chol ~ age + dbp + smoke
  Res.Df   RSS Df Sum of Sq    F Pr(>F)
1    2638 4676872
2    2639 4677574 -1      -702 0.40  0.53
3    2640 4681944 -1     -4370 2.46  0.12

```

The ANOVA model also suggests model A, for the following reasons:

- The  $p$  value of 0.233 indicates that moving from what we've called the kitchen sink model (model 1 in the ANOVA output) to what we've called model A (model 2 in the ANOVA output) does not have a statistically significant impact on predictive value.
- On the other hand, the  $p$  value of 0.016 indicates that moving from what we've called model A (model 2 in the ANOVA output) to what we've called model B (model 3 in the ANOVA output) does have a statistically significant impact on predictive value.
- Because these models are **nested** (model B is a proper subset of model A which is a proper subset of the kitchen sink) we can make these ANOVA comparisons directly.

```
BIC(model.kitchensink, model.A, model.B)
```

	df	BIC
model.kitchensink	7	27331
model.A	6	27323
model.B	5	27318

BIC disagrees, and prefers model B, since its BIC is smaller. The penalty for fitting additional predictors in BIC varies with the number of observations, and so (especially with larger samples) we can get meaningfully different AIC and BIC selections.

## 43.5 Comparing Models in the Test Sample (MSPE, MAPE)

Finally, we'll use our two candidate models (Model A and Model B) to predict the results in our holdout sample of 500 observations to see which model performs better in these new data (remember that our holdout sample was **not** used to identify or fit Models A or B.)

To do this, we first carefully specify the two models being compared

```
model.A <- lm(chol ~ age + bmi + dbp + smoke, data=wcgs.dev)
model.B <- lm(chol ~ age + dbp + smoke, data=wcgs.dev)
```

Next, use `predict` to make predictions for the test data:

```
modA.pre <- predict(model.A, newdata=wcgs.test)
modB.pre <- predict(model.B, newdata=wcgs.test)
```

Just to fix ideas, here are the first few predictions for Model A...

```
head(modA.pre)
```

```

1   2   3   4   5   6
223 226 222 216 220 234

```

We can compare these to the first few observed values of `chol` in the test sample.

```
head(wcgs.test$chol)
```

```
[1] 171 326 220 256 276 194
```

Next, calculate errors (observed value minus predicted value) for each model:

```
modA.err <- wcgs.test$chol - modA.pre
modB.err <- wcgs.test$chol - modB.pre
```

Again, just to be sure we understand, we look at the first few errors for Model A.

```
head(modA.err)
```

```
1      2      3      4      5      6
-52.24 99.99 -2.42 39.68 56.40 -39.68
```

Next, we calculate the absolute errors (as  $|observed - predicted|$ ) from each model in turn:

```
modA.abserr <- abs(modA.err)
modB.abserr <- abs(modB.err)
```

Let's look at the first few absolute errors for Model A.

```
head(modA.abserr)
```

```
1      2      3      4      5      6
52.24 99.99 2.42 39.68 56.40 39.68
```

Next, we calculate the squared prediction errors from each model in turn:

```
modA.sqerr <- modA.err^2
modB.sqerr <- modB.err^2
```

And again, we look at the first few squared errors for Model A.

```
head(modA.sqerr)
```

```
1      2      3      4      5      6
2729.26 9997.02 5.84 1574.86 3180.65 1574.34
```

To obtain our two key summaries: mean absolute prediction error and mean squared prediction error, I just use the `summary` function.

```
summary(modA.abserr)
```

Min.	1st Qu.	Median	Mean	3rd Qu.	Max.	NA's
0	12	26	33	45	423	2

```
summary(modB.abserr)
```

Min.	1st Qu.	Median	Mean	3rd Qu.	Max.	NA's
0	13	26	33	45	426	2

```
summary(modA.sqerr)
```

Min.	1st Qu.	Median	Mean	3rd Qu.	Max.	NA's
0	146	686	2083	1988	179119	2

```
summary(modB.sqerr)
```

Min.	1st Qu.	Median	Mean	3rd Qu.	Max.	NA's
0	157	682	2094	2031	181598	2

	Model	MAPE	MSPE
A (age + bmi + dbp + smoke)	32.92	2083	
B (age + dbp + smoke)	33.01	2094	

Note that smaller values on these metrics are better, so that each method (barely) selects Model A over Model B. The NAs you see above refer to two patients in the `wcgs` data with missing values on one or more of the variables included in our kitchen sink model. I absolutely should have identified that problem at the beginning, and either omitted those or done some imputation back at the start. I'll show that in the next section.

So, based on the test sample results, we slightly favor Model A.



# Chapter 44

## Dealing with Missing Data

So what we should have done at the start of our `wcgs` analysis was to identify any issues with missing values or whether any variables show unreasonably large or small values.

```
wcgs.s <- wcgs %>%  
  dplyr::select(id, chol, age, bmi, sbp, dbp, smoke)
```

### 44.1 Identifying Missingness

If you just want a count of missing values, you can use `colSums` and `is.na`

```
colSums(is.na(wcgs.s))
```

```
id  chol  age  bmi  sbp  dbp  smoke  
0    12    0    0    0    0    0
```

The `mice` package provides the `md.pattern` function to identify missingness patterns.

```
library(mice)  
md.pattern(wcgs.s)
```

```
      id age bmi sbp dbp smoke chol  
3142  1   1   1   1   1     1   1   0  
    12  1   1   1   1   1     1   0   1  
    0   0   0   0   0     0   12  12
```

Given the relatively small set of variables we're studying here, I would run the `describe` function from the `Hmisc` package on each variable (maybe skipping `id`) in order to identify missing and (potentially) unrealistic values through range checks.

```
Hmisc::describe(wcgs.s[-1]) # won't bother summarizing id
```

```
wcgs.s[-1]
```

```
6 Variables      3154 Observations  
-----  
chol  
  n  missing  distinct      Info      Mean      Gmd      .05      .10  
  3142      12      237       1  226.4    47.99   161.1   175.0  
  .25      .50      .75       .90     .95  
  197.2    223.0    253.0     280.0    302.0
```

lowest : 103 110 111 112 113, highest: 386 390 400 414 645

---

age

	n	missing	distinct	Info	Mean	Gmd	.05	.10
3154	0	21	0.996	46.28	6.256	39	39	40
.25	.50	.75	.90	.95				
42	45	50	55	57				

lowest : 39 40 41 42 43, highest: 55 56 57 58 59

---

bmi

	n	missing	distinct	Info	Mean	Gmd	.05	.10
3154	0	679	1	24.52	2.803	20.59	20.59	21.52
.25	.50	.75	.90	.95				
22.96	24.39	25.84	27.45	28.73				

lowest : 11.2 15.7 16.9 17.2 17.2, highest: 36.0 37.2 37.2 37.7 38.9

---

sbp

	n	missing	distinct	Info	Mean	Gmd	.05	.10
3154	0	62	0.996	128.6	16.25	110	110	112
.25	.50	.75	.90	.95				
120	126	136	148	156				

lowest : 98 100 102 104 106, highest: 200 208 210 212 230

---

dbp

	n	missing	distinct	Info	Mean	Gmd	.05	.10
3154	0	42	0.992	82.02	10.51	68	68	70
.25	.50	.75	.90	.95				
76	80	86	94	100				

lowest : 58 60 62 64 66, highest: 125 129 130 136 150

---

smoke

	n	missing	distinct
3154	0	2	

Value	No	Yes
Frequency	1652	1502
Proportion	0.524	0.476

---

No values are outside the range of plausibility, and in any case, we see that we have 12 missing chol values in the full data set. Options?

- We might choose to omit those rows before creating our test and training samples.
  - If we’re building a model where chol is the outcome, I would omit those cases, as (for 431, at least) I won’t impute outcomes. This is R’s default.
- We might choose to impute missing values after we partition.

## 44.2 Complete Case Analysis: A model for chol

Suppose we want to build a model for `chol` using age, body-mass index and systolic blood pressure, using the entire `wcgs` data frame, except those subjects with a missing `chol` value. Note that this is the default approach R takes, regardless of whether `chol` is used as an outcome or predictor.

```
model1 <- lm(chol ~ age + bmi + sbp, data = wcgs.s)
summary(model1)
```

```
Call:
lm(formula = chol ~ age + bmi + sbp, data = wcgs.s)
```

Residuals:

Min	1Q	Median	3Q	Max
-125.4	-28.6	-3.0	25.9	409.4

Coefficients:

	Estimate	Std. Error	t value	Pr(> t )							
(Intercept)	146.871	10.320	14.23	< 2e-16 ***							
age	0.561	0.141	3.98	7.2e-05 ***							
bmi	0.681	0.312	2.18	0.029 *							
sbp	0.287	0.054	5.31	1.2e-07 ***							
---											
Signif. codes:	0	'***'	0.001	'**'	0.01	'*'	0.05	'.'	0.1	' '	1

Residual standard error: 43 on 3138 degrees of freedom

(12 observations deleted due to missingness)

Multiple R-squared: 0.0214, Adjusted R-squared: 0.0205

F-statistic: 22.9 on 3 and 3138 DF, p-value: 1.16e-14

The notification under the residual standard error indicates that 12 observations (the 12 with missing `chol`, since no other variables in this group have missing data) were dropped before the model was fit. You can also tell this from the degrees of freedom.

- We have four coefficients to fit (intercept and slopes of `age`, `bmi` and `sbp`) so we have  $(4 - 1) = 3$  numerator degrees of freedom.
- The ANOVA F-statistic report also specifies 3138 denominator degrees of freedom.
- Total df is always one less than the total number of observations used in the model.
- That suggests we are using  $(3 + 3138) + 1 = 3142$  subjects in this model.
- But `wcgs.s` actually contains 3,154 people so 12 were omitted.

```
dim(wcgs.s)
```

```
[1] 3154    7
```

### 44.2.1 Removing all subjects with NA, via `na.omit`

So, if we want to fit a complete case analysis, we don't, technically, have to do anything except specify the model. We can also make this explicit by first pruning all subjects with any missing values on any variables from our tibble.

```
wcgs.subnoNA <- dplyr::select(wcgs, id, chol, age, bmi, sbp) %>%
  na.omit ## the na.omit function drops all cases with NA
dim(wcgs.subnoNA)
```

```
[1] 3142    5
```

### 44.2.2 Removing subjects with NA on one particular variable

If we wanted to specify that only subjects with missing `chol` should be removed from the data set (in case there were missing values in other variables), we could use the following approach.

Here, I'll work with a data set (`wcgs.s3`) that contains 12 missing `chol` values, and 2 missing `arcus` values, and build a new set (`wcgs.s3noNACHOL`) that retains the subjects who have a `chol` value, regardless of whether they are also missing `arcus`, but not include the 12 subjects with missing `chol` data

```
wcgs.s3 <- dplyr::select(wcgs, id, chol, arcus)
md.pattern(wcgs.s3)
```

	<code>id</code>	<code>arcus</code>	<code>chol</code>
3140	1	1	1 0
12	1	1	0 1
2	1	0	1 1
0	2	12	14

```
wcgs.s3noNACHOL <- wcgs.s3 %>%
  filter(!is.na(chol))
md.pattern(wcgs.s3noNACHOL)
```

	<code>id</code>	<code>chol</code>	<code>arcus</code>
3140	1	1	1 0
2	1	1	0 1
0	0	2	2

## 44.3 Using Multiple Imputation to fit our Regression Model

Following the approach we outlined in the R-20 document<sup>1</sup>, we'll try fitting the model while imputing the 12 cholesterol values, 100 times each, creating 100 new "complete" data sets.

```
wcgs.sub <- wcgs %>% dplyr::select(id, chol, age, bmi, sbp)
chol.mi <- mice(wcgs.sub, m = 100, maxit = 5,
                 meth = "pmm", printFlag = FALSE, seed = 4314)
```

### 44.3.1 Examining a Single Imputed Data Set

We could look at any one of our 100 imputations in some detail, perhaps the 12th such imputation, as follows:

```
imp12 <- mice::complete(chol.mi, 12)
Hmisc::describe(imp12$chol)
```

<code>n</code>	<code>missing</code>	<code>distinct</code>	<code>Info</code>	<code>Mean</code>	<code>Gmd</code>	<code>.05</code>	<code>.10</code>
3154	0	237	1	226.4	47.95	161	175
.25	.50	.75	.90	.95			
198	223	253	280	302			

```
lowest : 103 110 111 112 113, highest: 386 390 400 414 645
```

---

<sup>1</sup>Also see <https://stat.ethz.ch/education/seminsters/ss2012/ams/paper/mice.pdf>

Working with a particular imputation might allow us to produce plots of the imputed data, or of diagnostics after a regression model, but we'll focus our interpretation of regression results (in 431) on estimates of coefficients and of things like  $R^2$ . For that, we want to use all 100 imputations, in a pooled approach.

### 44.3.2 Fitting a Pooled Regression Model across the Imputations

Next, we'll fit a set of pooled regression model estimates across all 100 imputations, producing the following results. We'll even estimate both forms of  $R^2$ .

```
model.1mi <- with(chol.mi, lm(chol ~ age + bmi + sbp))
pool(model.1mi)
```

Call: pool(object = model.1mi)

Pooled coefficients:

	age	bmi	sbp
(Intercept)	147.047	0.564	0.686
			0.283

Fraction of information about the coefficients missing due to nonresponse:

	age	bmi	sbp
(Intercept)	0.00795	0.00835	0.00694
			0.01739

```
round(summary(pool(model.1mi)), 2)
```

	est	se	t	df	Pr(> t )	lo	95	hi	95	nmis	fmi
(Intercept)	147.05	10.33	14.24	3120	0.00	126.80	167.29		NA	0.01	
age	0.56	0.14	4.00	3118	0.00	0.29	0.84		0	0.01	
bmi	0.69	0.31	2.20	3124	0.03	0.07	1.30		0	0.01	
sbp	0.28	0.05	5.23	3068	0.00	0.18	0.39		0	0.02	
	lambda										
(Intercept)	0.01										
age	0.01										
bmi	0.01										
sbp	0.02										

```
pool.r.squared(model.1mi, adjusted = FALSE)
```

	est	lo	95	hi	95	fmi
R^2	0.0213	0.0124	0.0324	0.00899		

```
pool.r.squared(model.1mi, adjusted = TRUE)
```

	est	lo	95	hi	95	fmi
adj R^2	0.0204	0.0117	0.0313	0.00939		

- fmi in the output above contains the fraction of missing information.
- lambda in that output is the proportion of the total variance that is attributable to the missing data.

So, how do these estimates after imputation compare to our complete case analysis originally developed as model1, and re-summarized below?

```
summary(model1)
```

Call:  
`lm(formula = chol ~ age + bmi + sbp, data = wcgs.s)`

Residuals:

```

      Min      1Q Median      3Q      Max
-125.4   -28.6   -3.0    25.9   409.4

Coefficients:
            Estimate Std. Error t value Pr(>|t|)
(Intercept) 146.871    10.320   14.23 < 2e-16 ***
age          0.561     0.141    3.98  7.2e-05 ***
bmi          0.681     0.312    2.18   0.029 *
sbp          0.287     0.054    5.31  1.2e-07 ***
---
Signif. codes:  0 '***' 0.001 '**' 0.01 '*' 0.05 '.' 0.1 ' ' 1

Residual standard error: 43 on 3138 degrees of freedom
(12 observations deleted due to missingness)
Multiple R-squared:  0.0214, Adjusted R-squared:  0.0205
F-statistic: 22.9 on 3 and 3138 DF, p-value: 1.16e-14

```

## 44.4 Comparing Two Models After Imputation with `pool.compare`

Suppose we want to assess whether a model for `chol` with `age`, `bmi` and `sbp` is statistically significantly more effective than a model with `age` alone. As long as the models we want to compare are *nested* (so that one model is a proper subset of the other), then this can be done as follows, to produce a Wald test.

```

model.1mi <- with(chol.mi, lm(chol ~ age + bmi + sbp))
model.2mi <- with(chol.mi, lm(chol ~ age))
comp1 <- pool.compare(model.1mi, model.2mi, method = "Wald")

comp1$qbar1 # pooled estimate of first (larger) model

(Intercept)       age       bmi       sbp
  147.047      0.564      0.686      0.283

comp1$qbar0 # pooled estimate of second (smaller) model

(Intercept)       age
  193.935      0.701

comp1$Dm # Wald test statistic comparing the models

[1] 21.2

comp1$df1; comp1$df2 # degrees of freedom for Wald test

[1] 2

[1] 1403228

comp1$pvalue # p value for Wald test

[1] 6.04e-10

```

Practically, a significant value of the Wald test here suggests that the difference in predictive value between `model.1mi` and `model.2mi` is statistically significant, and thus, that you need the larger model. A non-significant Wald test would be consistent with a situation where you could use the model with `age` alone.

Here, we clearly want to retain `bmi` and `sbp` as compared to dropping them both from the model. This isn't a surprise, as we saw previously that *both* `bmi` and `sbp` had apparent predictive value (by the t test) even after all other variables were already in the model.

To create the overall F test, we could compare our model to an intercept only model, `model.intmi <- with(chol.mi, lm(chol ~ 1))` but we'll skip that here.



# Chapter 45

## BMI and Employment: Working with Categorical Predictors

### 45.1 The Data

A study recently published in *BMJ Open* looked at the differential relationship between employment status and body-mass index among middle-aged and elderly adults living in South Korea<sup>1</sup>. Data from this study were available online thanks to the Dryad data package<sup>2</sup>. The original data came from a nationally representative sample of 7228 participants in the Korean Longitudinal Study of Aging. I sampled these data, and did some data “rectangling” (wrangling) to build the `emp_bmi.csv` file on our web site.

The available data in `emp_bmi` describe 999 subjects, and included are 8 variables:

Variable	Description	NA?
<code>pid</code>	subject identification number (categorical)	0
<code>bmi</code>	our outcome, quantitative, body-mass index	0
<code>age</code>	subject's age (between 51 and 95)	1
<code>gender</code>	subject's gender (male or female)	0
<code>employed</code>	employment status indicator (1/0)	1
<code>married</code>	marital status indicator (1/0)	1
<code>alcohol</code>	3-level factor	2
<code>education</code>	4-level factor	5

```
Hmisc::describe(emp_bmi)
```

```
emp_bmi

 8 Variables      999 Observations
-----
pid
  n  missing distinct      Info      Mean      Gmd       .05       .10
```

<sup>1</sup>See Noh J, Kim J, Park J, Oh I, Kwon YD (2016) Age and gender differential relationship between employment status and body mass index among middle-aged and elderly adults: a cross-sectional study. *BMJ Open* 6(11): e012117. <http://dx.doi.org/10.1136/bmjopen-2016-012117>

<sup>2</sup>Noh J, Kim J, Park J, Oh I, Kwon YD (2016) Data from: Age and gender differential relationship between employment status and body mass index among middle aged and elderly adults: a cross-sectional study. Dryad Digital Repository. <http://dx.doi.org/10.5061/dryad.ng8mn>

999	0	999	1	31774	20178	3506	6961
.25	.50	.75	.90	.95			
16671	31761	46902	54635	58433			
<hr/>							
lowest : 22 41 52 82 112, highest: 61471 61481 61532 61641 61691							
<hr/>							
bmi							
n	missing	distinct	Info	Mean	Gmd	.05	.10
999	0	373	1	23.24	2.808	19.21	20.13
.25	.50	.75	.90	.95			
21.54	23.24	24.78	26.56	27.34			
<hr/>							
lowest : 15.6 15.6 16.2 16.4 16.5, highest: 31.1 31.2 32.0 33.5 34.7							
<hr/>							
age							
n	missing	distinct	Info	Mean	Gmd	.05	.10
998	1	43	0.999	66.29	11.56	52	53
.25	.50	.75	.90	.95			
57	66	74	81	83			
<hr/>							
lowest : 51 52 53 54 55, highest: 89 90 92 93 95							
<hr/>							
gender							
n	missing	distinct					
999	0	2					
<hr/>							
Value female male							
Frequency	570	429					
Proportion	0.571	0.429					
<hr/>							
employed							
n	missing	distinct	Info	Sum	Mean	Gmd	
998	1	2	0.723	404	0.4048	0.4824	
<hr/>							
married							
n	missing	distinct	Info	Sum	Mean	Gmd	
998	1	2	0.548	758	0.7595	0.3657	
<hr/>							
alcohol							
n	missing	distinct					
997	2	3					
<hr/>							
Value alcohol dependent heavy drinker							
Frequency			45			306	
Proportion			0.045			0.307	
<hr/>							
Value normal drinker or non-drinker							
Frequency			646				
Proportion			0.648				
<hr/>							
education							
n	missing	distinct					

994	5	4						
Value	1 elem school grad or lower		2 middle school grad					
Frequency		421		180				
Proportion		0.424		0.181				
Value	3 high school grad	4 college grad or higher						
Frequency	292		101					
Proportion	0.294		0.102					

### 45.1.1 Specifying Outcome and Predictors for our Model

In the original study, a key goal was to understand the relationship between employment and body-mass index. Our goal in this example will be to create a model to predict `bmi` focusing on employment status (so our key predictor is `employed`) while accounting for the additional predictors `age`, `gender`, `married`, `alcohol` and `education`. A natural thing to do would be to consider interactions of these predictor variables (for example, does the relationship between `bmi` and `employed` change when comparing men to women?) but we'll postpone that discussion until 432.

### 45.1.2 Dealing with Missing Predictor Values

```
md.pattern(emp_bmi)
```

	pid	bmi	gender	age	employed	married	alcohol	education	
990	1	1	1	1	1	1	1	1	0
	1	1	1	1	1	0	1	1	1
	1	1	1	1	1	1	0	1	1
	1	1	1	1	1	1	1	0	1
	5	1	1	1	1	1	1	0	1
	1	1	1	1	0	1	1	0	1
	0	0	0	1	1	1	2	5	10

We will eventually build a model to predict `bmi` using all of the other variables besides `pid`. So we'll eventually have to account for the 9 people with missing values (one of whom has two missing values, as we see above.) What I'm going to do in this example is to first build a complete-case analysis on the 990 subjects without missing values and then, later, do multiple imputation to account for the 9 subjects with missing values (and their 10 actual missing values) sensibly.

I'll put the "complete cases" data set of 990 subjects in `emp_bmi_noNA`.

```
emp_bmi_noNA <- emp_bmi %>% na.omit  
emp_bmi_noNA
```

# A tibble: 990 x 8	pid	bmi	age	gender	employed	married	alcohol
	<int>	<dbl>	<int>	<fctr>	<int>	<int>	<fctr>
1	22	20.8	58	male	1	1	heavy drinker
2	41	21.4	76	female	0	1	normal drinker or non-drinker
3	52	20.9	66	female	1	1	heavy drinker
4	82	23.7	67	male	1	1	alcohol dependent
5	112	25.5	63	female	0	1	heavy drinker
6	181	20.5	51	female	1	1	heavy drinker
7	182	25.3	51	male	1	1	alcohol dependent

```

8   411  20.8    66 female      1      1 normal drinker or non-drinker
9   491  20.8    64 female      0      1 normal drinker or non-drinker
10  531  24.3    84 female      0      0 normal drinker or non-drinker
# ... with 980 more rows, and 1 more variables: education <fctr>
colSums(is.na(emp_bmi_noNA))

  pid      bmi     age gender employed married alcohol
  0       0       0      0      0       0       0      0
education
  0

```

## 45.2 The “Kitchen Sink” Model

A “kitchen sink” model includes all available predictors.

```

ebmodel.1 <- lm(bmi ~ age + gender + employed + married +
                  alcohol + education, data = emp_bmi_noNA)
summary(ebmodel.1)

```

Call:

```
lm(formula = bmi ~ age + gender + employed + married + alcohol +
   education, data = emp_bmi_noNA)
```

Residuals:

Min	1Q	Median	3Q	Max
-7.703	-1.606	-0.049	1.499	11.730

Coefficients:

	Estimate	Std. Error	t value	Pr(> t )		
(Intercept)	26.1539	0.8836	29.60	< 2e-16		
age	-0.0426	0.0107	-4.00	6.9e-05		
gendermale	0.2981	0.2027	1.47	0.142		
employed	-0.4576	0.1915	-2.39	0.017		
married	0.0944	0.2128	0.44	0.657		
alcoholheavy drinker	0.2532	0.4073	0.62	0.534		
alcoholnormal drinker or non-drinker	0.1412	0.4077	0.35	0.729		
education2 middle school grad	-0.2886	0.2402	-1.20	0.230		
education3 high school grad	-0.5012	0.2219	-2.26	0.024		
education4 college grad or higher	-0.7986	0.3107	-2.57	0.010		
(Intercept)	***					
age	***					
gendermale						
employed	*					
married						
alcoholheavy drinker						
alcoholnormal drinker or non-drinker						
education2 middle school grad						
education3 high school grad	*					
education4 college grad or higher	*					
---						
Signif. codes:	0 '***'	0.001 '**'	0.01 '*'	0.05 '.'	0.1 ' '	1

```
Residual standard error: 2.51 on 980 degrees of freedom
Multiple R-squared:  0.0225,    Adjusted R-squared:  0.0135
F-statistic: 2.51 on 9 and 980 DF,  p-value: 0.00772
```

## 45.3 Using Categorical Variables (Factors) as Predictors

We have six predictors here, and five of them are categorical. Note that R recognizes each kind of variable in this case and models them appropriately. Let's look at the coefficients of our model.

### 45.3.1 gender: A binary variable represented by letters

The `gender` variable contains the two categories: male and female, and R recognizes this as a factor. When building a regression model with such a variable, R assigns the first of the two levels of the factor to the baseline, and includes in the model an indicator variable for the second level. By default, R assigns each factor a level order alphabetically.

So, in this case, we have:

```
is.factor(emp_bmi_noNA$gender)
```

```
[1] TRUE
```

```
levels(emp_bmi_noNA$gender)
```

```
[1] "female" "male"
```

As you see in the model, the `gender` information is captured by the indicator variable `gendermale`, which is 1 when `gender = male` and 0 otherwise.

So, when our model includes:

```
Coefficients: Estimate Std. Error t value Pr(>|t|)
gendermale      0.29811   0.20271   1.471   0.1417
```

this means that a male subject is predicted to have an outcome that is 0.29811 points higher than a female subject, if they have the same values of all of the other predictors.

Note that if we wanted to switch the levels so that “male” came first (and so that R would use “male” as the baseline category and “female” as the 1 value in an indicator), we could do so with the `forcats` package and the `fct_relevel` command. Building a model with this version of `gender` will simply reverse the sign of our indicator variable, but not change any of the other output.

```
emp_bmi_noNA$gender.2 <- fct_relevel(emp_bmi_noNA$gender, "male", "female")
revised.model <- lm(bmi ~ age + gender.2 + employed + married +
                     alcohol + education, data = emp_bmi_noNA)
summary(revised.model)
```

Call:

```
lm(formula = bmi ~ age + gender.2 + employed + married + alcohol +
  education, data = emp_bmi_noNA)
```

Residuals:

Min	1Q	Median	3Q	Max
-7.703	-1.606	-0.049	1.499	11.730

Coefficients:

	Estimate	Std. Error	t value	Pr(> t )
(Intercept)	26.4520	0.9465	27.95	< 2e-16
age	-0.0426	0.0107	-4.00	6.9e-05
gender.2female	-0.2981	0.2027	-1.47	0.142
employed	-0.4576	0.1915	-2.39	0.017
married	0.0944	0.2128	0.44	0.657
alcoholheavy drinker	0.2532	0.4073	0.62	0.534
alcoholnormal drinker or non-drinker	0.1412	0.4077	0.35	0.729
education2 middle school grad	-0.2886	0.2402	-1.20	0.230
education3 high school grad	-0.5012	0.2219	-2.26	0.024
education4 college grad or higher	-0.7986	0.3107	-2.57	0.010
(Intercept)	***			
age	***			
gender.2female				
employed	*			
married				
alcoholheavy drinker				
alcoholnormal drinker or non-drinker				
education2 middle school grad				
education3 high school grad	*			
education4 college grad or higher	*			
---				
Signif. codes:	0 *** 0.001 ** 0.01 * 0.05 . 0.1 ' ' 1			

Residual standard error: 2.51 on 980 degrees of freedom  
 Multiple R-squared: 0.0225, Adjusted R-squared: 0.0135  
 F-statistic: 2.51 on 9 and 980 DF, p-value: 0.00772

Note that the two categories here need to be both *mutually exclusive* (a subject cannot be in more than one category) and *collectively exhaustive* (all subjects must fit into this set of categories) in order to work properly as a regression predictor.

### 45.3.2 employed: A binary variable represented a 1/0 indicator

The `employed` and `married` variables are each described using an indicator variable, which is 1 if the condition of interest holds and 0 if it does not. R doesn't recognize this as a factor, but rather as a quantitative variable. However, this is no problem for modeling, where we just need to remember that if `employed` = 1, the subject is employed, and if `employed` = 0, the subject is not employed, to interpret the results. The same approach is used for `married`.

Coefficients:	Estimate	Std. Error	t value	Pr(> t )
employed	-0.45761	0.19153	-2.389	0.0171 *
married	0.09438	0.21280	0.444	0.6575

So, in our model, if subject A is employed, they are expected to have an outcome that is 0.46 points lower (-0.46 points higher) than subject B who is not employed but otherwise identical to subject A.

Similarly, if subject X is married, and subject Y is unmarried, but they otherwise have the same values of all predictors, then our model will predict a `bmi` for X that is 0.094 points higher than for Y.

### 45.3.3 alcohol: A three-category variable coded by names

Our `alcohol` information divides subjects into three categories, which are:

- normal drinker or non-drinker
- heavy drinker
- alcohol dependent

R builds a model using  $k - 1$  predictors to describe a variable with  $k$  levels. As mentioned previously, R selects a baseline category when confronted with a factor variable, and it always selects the first level as the baseline. The levels are sorted alphabetically, unless we tell R to sort them some other way. So, we have

Coefficients:	Estimate	Std. Error	t value	Pr(> t )
alcoholheavy drinker	0.25317	0.40727	0.622	0.5343
alcoholnormal drinker or non-drinker	0.14121	0.40766	0.346	0.7291

How do we interpret this?

- Suppose subject A is alcohol dependent, B is a heavy drinker and C is a normal drinker or non-drinker, but subjects A-C have the same values of all other predictors.
- Our model predicts that B would have a BMI that is 0.25 points higher than A.
- Our model predicts that C would have a BMI that is 0.14 points higher than A.

A good way to think about this...

Subject	Status	alcoholheavy drinker	alcoholnormal drinker or non-drinker
A	alcohol dependent	0	0
B	heavy drinker	1	0
C	normal drinker or non-drinker	0	1

and so, with two variables, we cover each of these three possible `alcohol` levels.

When we have an ordered variable like this one, we usually want the baseline category to be at either end of the scale (either the highest or the lowest, but not something in the middle.) Another good idea in many settings is to use as the baseline category the most common category. Here, the baseline R chose was “alcohol dependent” which is the least common category, so I might want to use the `fct_relevel` function again to force R to choose, say, normal drinker/non-drinker as the baseline category.

```
emp_bmi_noNA$alcohol.2 <- fct_relevel(emp_bmi_noNA$alcohol,
                                         "normal drinker or non-drinker", "heavy drinker")
revised.model.2 <- lm(bmi ~ age + gender + employed + married +
                      alcohol.2 + education, data = emp_bmi_noNA)
summary(revised.model.2)
```

Call:

```
lm(formula = bmi ~ age + gender + employed + married + alcohol.2 +
   education, data = emp_bmi_noNA)
```

Residuals:

Min	1Q	Median	3Q	Max
-7.703	-1.606	-0.049	1.499	11.730

Coefficients:

Coefficients:	Estimate	Std. Error	t value	Pr(> t )
(Intercept)	26.2951	0.8286	31.73	< 2e-16 ***
age	-0.0426	0.0107	-4.00	6.9e-05 ***

```

gendermale          0.2981    0.2027    1.47    0.142
employed           -0.4576    0.1915   -2.39    0.017 *
married            0.0944    0.2128    0.44    0.657
alcohol.2heavy drinker  0.1120    0.1965    0.57    0.569
alcohol.2alcohol dependent -0.1412    0.4077   -0.35    0.729
education2 middle school grad -0.2886    0.2402   -1.20    0.230
education3 high school grad   -0.5012    0.2219   -2.26    0.024 *
education4 college grad or higher -0.7986    0.3107   -2.57    0.010 *
---
Signif. codes:  0 '***' 0.001 '**' 0.01 '*' 0.05 '.' 0.1 ' ' 1

```

Residual standard error: 2.51 on 980 degrees of freedom  
 Multiple R-squared: 0.0225, Adjusted R-squared: 0.0135  
 F-statistic: 2.51 on 9 and 980 DF, p-value: 0.00772

How do we interpret this revised model?

- Again, subject A is alcohol dependent, B is a heavy drinker and C is a normal drinker or non-drinker, but subjects A-C have the same values of all other predictors.
- Our model predicts that B would have a BMI that is 0.11 points higher than C.
- Our model predicts that A would have a BMI that is 0.14 points lower than C.

So, those are the same conclusions, just rephrased.

#### 45.3.4 t tests and multi-categorical variables

The usual “last predictor in” t test works perfectly for binary factors, but suppose we have a factor like `alcohol` which is represented by two different indicator variables. If we want to know whether the `alcohol` information, as a group, adds statistically significant value to the model that includes all of the other predictors, then our best strategy is to compare two models - one with the alcohol information, and one without.

```

model.with.a <- lm(bmi ~ age + gender + alcohol + employed + married + education,
                     data = emp_bmi_noNA)
model.no.a <- lm(bmi ~ age + gender + employed + married + education,
                  data = emp_bmi_noNA)
anova(model.with.a, model.no.a)

```

#### Analysis of Variance Table

```

Model 1: bmi ~ age + gender + alcohol + employed + married + education
Model 2: bmi ~ age + gender + employed + married + education
  Res.Df  RSS Df Sum of Sq   F Pr(>F)
1     980 6190
2     982 6194 -2      -3.63 0.29   0.75

```

The *p* value for both of the indicator variables associated with `alcohol` combined is 0.75, according to an ANOVA F test with 2 degrees of freedom.

Note that we can get the same information from an ANOVA table of the larger model if we add the `alcohol` predictor to the model last.

```

anova(lm(bmi ~ age + gender + employed + married + education + alcohol,
          data = emp_bmi_noNA))

```

#### Analysis of Variance Table

```
Response: bmi
          Df Sum Sq Mean Sq F value Pr(>F)
age         1    56    55.5   8.79 0.0031 **
gender      1     0     0.4   0.06 0.8090
employed    1    31    30.7   4.87 0.0276 *
married     1     0     0.4   0.06 0.8077
education   3    52    17.3   2.74 0.0422 *
alcohol      2     4     1.8   0.29 0.7504
Residuals  980  6190    6.3
---
Signif. codes:  0 '***' 0.001 '**' 0.01 '*' 0.05 '.' 0.1 ' ' 1
```

Again, we see  $p$  for the two alcohol indicators is 0.75.

### 45.3.5 education: A four-category variable coded by names

The `education` variable's codes are a little better designed. By preceding the text with a number for each code, we force R to attend to the level order we want to see.

Coefficients:	Estimate	Std. Error	t value	Pr(> t )
<code>education2 middle school grad</code>	-0.28862	0.24020	-1.202	0.2298
<code>education3 high school grad</code>	-0.50123	0.22192	-2.259	0.0241 *
<code>education4 college grad or higher</code>	-0.79862	0.31068	-2.571	0.0103 *

Since we have four education levels, we need those three indicator variables.

- `education2 middle school grad` is 1 if the subject is a middle school graduate, and 0 if they have some other status
- `education3 high school grad` is 1 if the subject is a high school graduate, and 0 if they have some other status
- `education4 college grad or higher` is 1 if the subject is a college graduate or has more education, and 0 if they have some other status.
- So the subjects with only elementary school or lower education are represented by zeros in all three indicators.

Suppose we have four subjects now, with the same values of all other predictors, but different levels of education.

Subject	Education	Estimated BMI
A	elementary school or less	A
B	middle school grad	A - 0.289
C	high school grad	A - 0.501
D	college grad	A - 0.799

Note that the four categories are *mutually exclusive* (a subject cannot be in more than one category) and *collectively exhaustive* (all subjects must fit into this set of categories.) As we have seen, this is a requirement of categorical variables in a regression analysis.

Let's run the ANOVA test for the `education` information captured in those three indicator variables...

```
anova(lm(bmi ~ age + gender + employed + married + alcohol + education,
        data = emp_bmi_noNA))
```

#### Analysis of Variance Table

Response: bmi

```

Df Sum Sq Mean Sq F value Pr(>F)
age      1    56    55.5   8.79 0.0031 **
gender    1     0     0.4   0.06 0.8090
employed  1    31    30.7   4.87 0.0276 *
married   1     0     0.4   0.06 0.8077
alcohol    2     4     1.8   0.28 0.7581
education  3    52    17.4   2.75 0.0418 *
Residuals 980  6190    6.3
---
Signif. codes:  0 '***' 0.001 '**' 0.01 '*' 0.05 '.' 0.1 ' ' 1

```

So, as a group, the three indicator variables add statistically significant predictive value at the 5% significance level, since the F test for those three variables has  $p = 0.042$

### 45.3.6 Interpreting the Kitchen Sink Model

So, again, here's our model, now pandered into a prettier format.

```

ebmodel.1 <- lm(bmi ~ age + gender + employed + married +
                  alcohol + education, data = emp_bmi_noNA)
pander(ebmodel.1)

```

Table 45.4: Fitting linear model:  $\text{bmi} \sim \text{age} + \text{gender} + \text{employed}$   
+  $\text{married} + \text{alcohol} + \text{education}$

	Estimate	Std. Error	t value	Pr(> t )
(Intercept)	26.15	0.8836	29.6	4.401e-138
age	-0.0426	0.01065	-3.998	6.858e-05
gendermale	0.2981	0.2027	1.471	0.1417
employed	-0.4576	0.1915	-2.389	0.01707
married	0.09438	0.2128	0.4435	0.6575
alcoholheavy drinker	0.2532	0.4073	0.6216	0.5343
alcoholnormal drinker or non-drinker	0.1412	0.4077	0.3464	0.7291
education2 middle school grad	-0.2886	0.2402	-1.202	0.2298
education3 high school grad	-0.5012	0.2219	-2.259	0.02413
education4 college grad or higher	-0.7986	0.3107	-2.571	0.0103

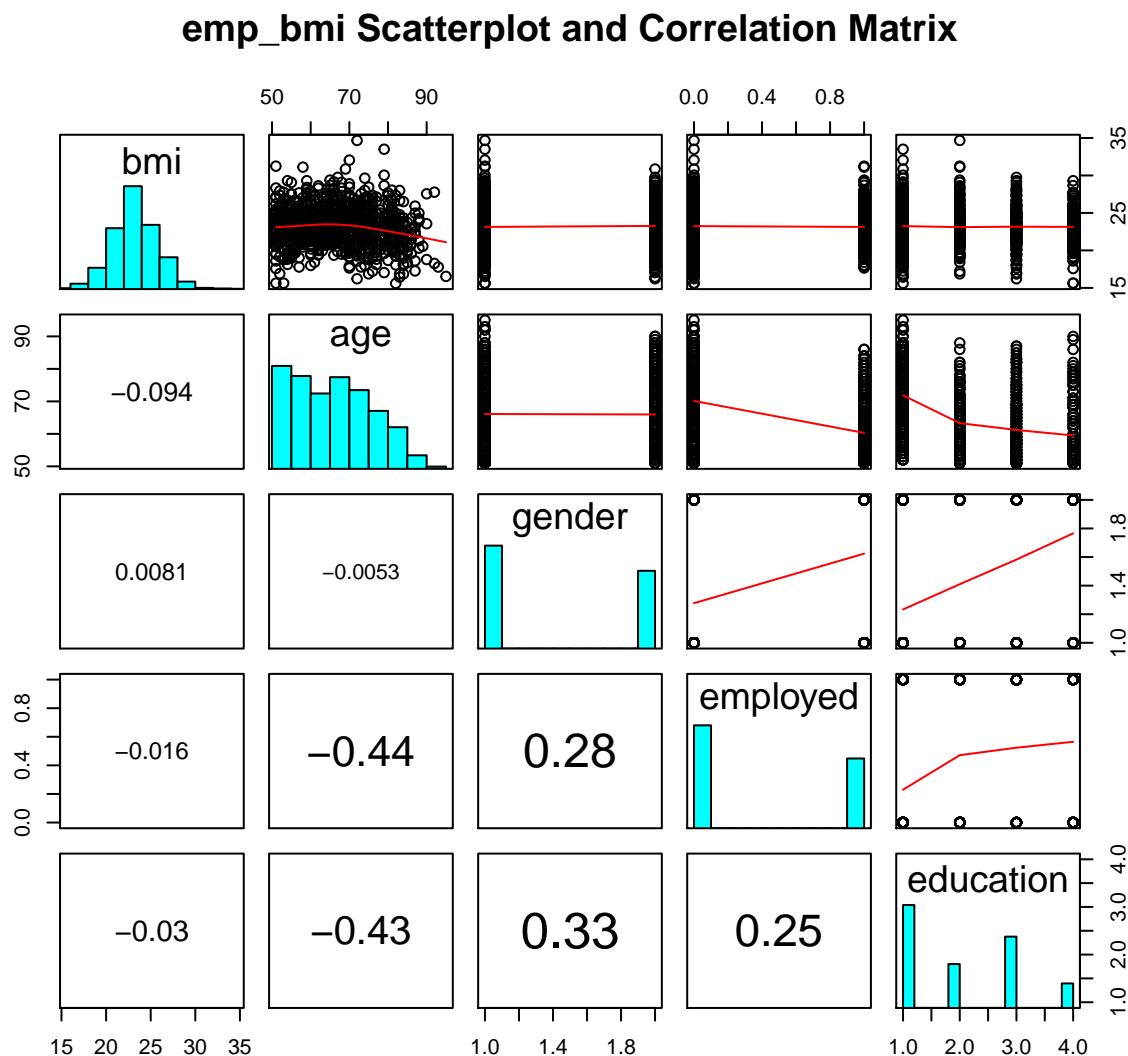
If we wanted to predict a BMI level for a new subject like the ones used in the development of this model, that prediction would be:

- 26.15
- minus 0.426 times the subject's `age`
- plus 0.298 if the subject's `gender` was `male`
- minus 0.458 if the subject's employment status was `employed`
- plus 0.253 if the subject's `alcohol` classification was `heavy drinker`
- plus 0.141 if the subject's `alcohol` classification was `normal drinker` or `non-drinker`
- minus 0.289 if the subject's `education` classification was 2 `middle school grad`
- minus 0.501 if the subject's `education` classification was 3 `high school grad`
- minus 0.799 if the subject's `education` classification was 4 `college grad` or `higher`

## 45.4 Scatterplot Matrix with Categorical Predictors

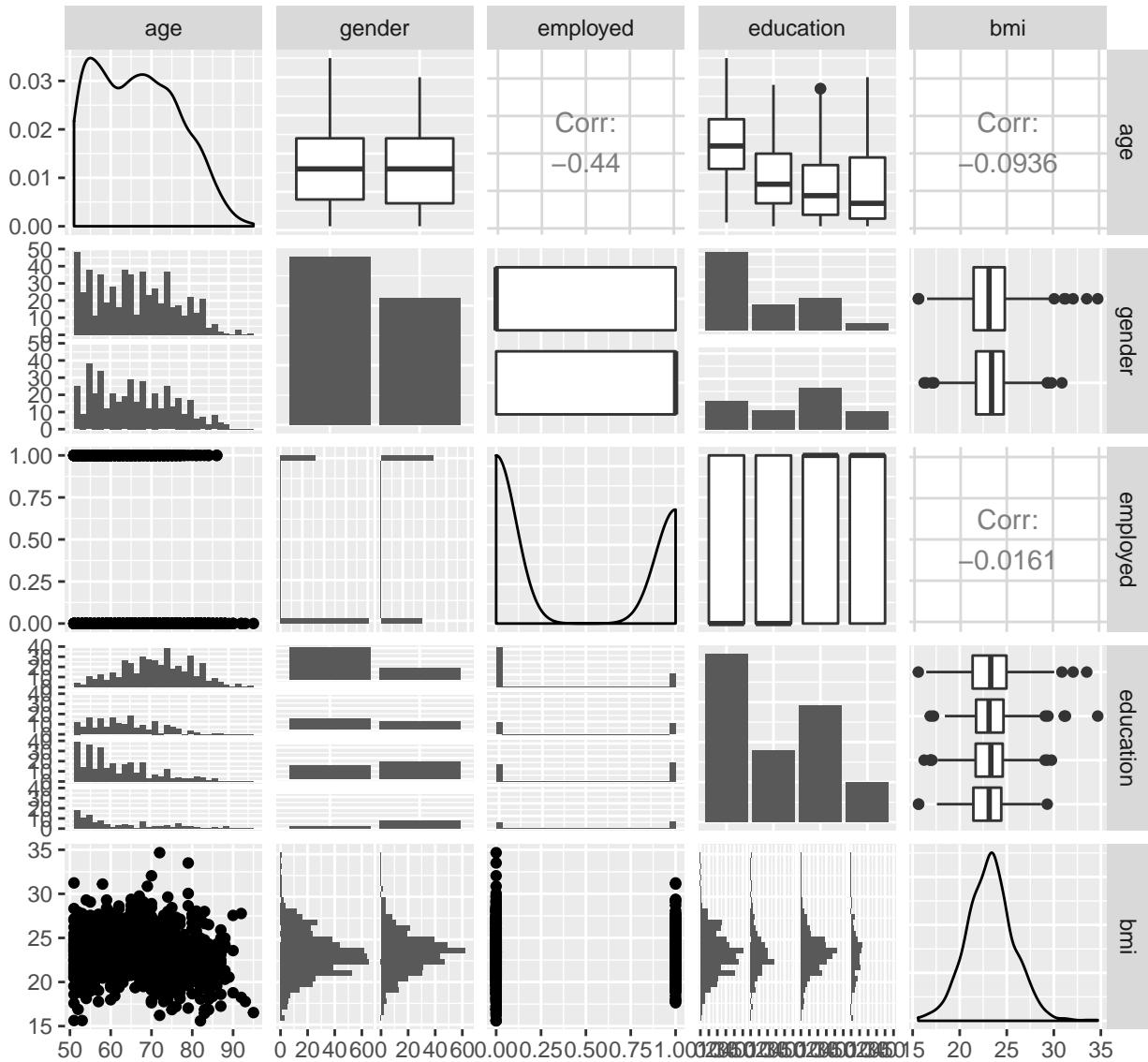
Let's look at a scatterplot matrix of a few key predictors, with my favorite approach (at least for quantitative predictors)...

```
pairs(~ bmi + age + gender + employed + education, data = emp_bmi_noNA,
      main = "emp_bmi Scatterplot and Correlation Matrix",
      upper.panel = panel.smooth,
      diag.panel = panel.hist,
      lower.panel = panel.cor)
```



Notice how the `ggpairs` approach reacts to the inclusion of factors as predictors...

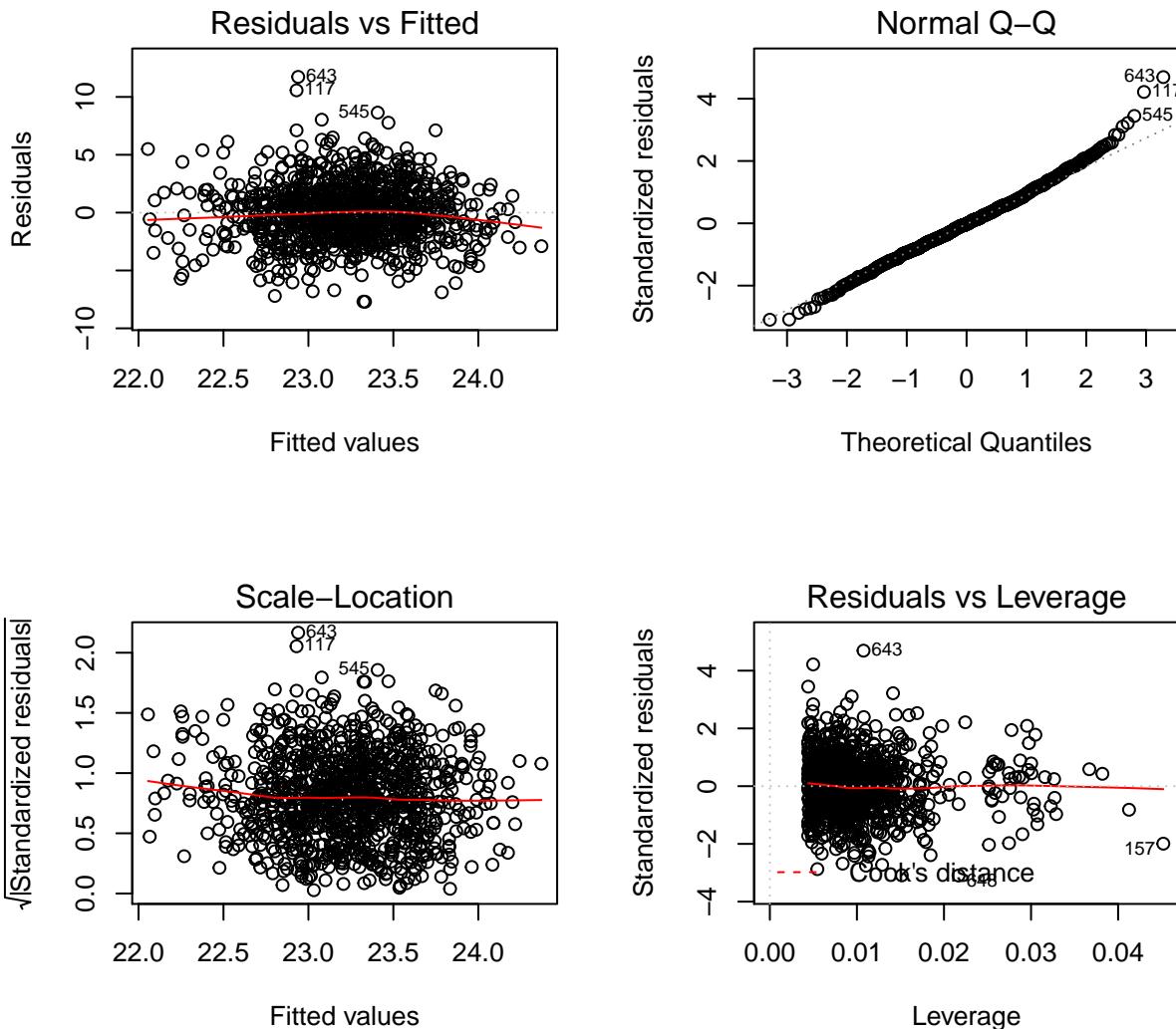
```
GGally::ggpairs(dplyr::select(emp_bmi_noNA, age, gender, employed, education, bmi))
```



## 45.5 Residual Plots when we have Categorical Predictors

Here are the main residual plots from the kitchen sink model `ebmodel.1` defined previously.

```
par(mfrow=c(2,2))
plot(ebmodel.1)
```



```
par(mfrow=c(1,1))
```

Sometimes, in small samples, the categorical variables will make the regression residuals line up in somewhat strange patterns. But in this case, there's no real problem. The use of categorical variables also has some impact on leverage, as it's hard for a subject to be a serious outlier in terms of a predictor if that predictor only has a few possible levels.

## 45.6 Stepwise Regression and Categorical Predictors

When R does backwards elimination for stepwise model selection, it makes decisions about each categorical variable as in/out across all of the indicator variables simultaneously, as you'd hope.

```
step(ebmodel.1)
```

```
Start: AIC=1835
bmi ~ age + gender + employed + married + alcohol + education
```

	Df	Sum of Sq	RSS	AIC
- alcohol	2	3.6	6194	1831
- married	1	1.2	6191	1833
<none>			6190	1835
- gender	1	13.7	6204	1835
- education	3	52.1	6242	1837
- employed	1	36.1	6226	1838
- age	1	101.0	6291	1849

Step: AIC=1831

bmi ~ age + gender + employed + married + education

	Df	Sum of Sq	RSS	AIC
- married	1	1.0	6195	1829
<none>			6194	1831
- gender	1	19.1	6213	1832
- education	3	51.9	6245	1833
- employed	1	34.7	6228	1835
- age	1	108.6	6302	1846

Step: AIC=1829

bmi ~ age + gender + employed + education

	Df	Sum of Sq	RSS	AIC
<none>		6195	1829	
- gender	1	22.2	6217	1831
- education	3	51.3	6246	1832
- employed	1	34.4	6229	1833
- age	1	125.4	6320	1847

Call:

`lm(formula = bmi ~ age + gender + employed + education, data = emp_bmi_noNA)`

Coefficients:

(Intercept)		age
	26.5021	-0.0446
gendermale		employed
	0.3406	-0.4457
education2 middle school grad		education3 high school grad
	-0.2879	-0.4977
education4 college grad or higher		
	-0.7904	

Note that the stepwise approach first drops two degrees of freedom (two indicator variables) for `alcohol` and then drops the one degree of freedom for `married` before it settles on a model with `age`, `gender`, `education` and `employed`.

## 45.7 Pooling Results after Multiple Imputation

As mentioned earlier, having built a model using complete cases, we should probably investigate the impact of multiple imputation on the missing observations. We'll fit 100 imputations using the `emp_bmi` data and then fit a pooled regression model across those imputations.

```
emp_bmi_mi <- mice(emp_bmi, m = 100, maxit = 5,
                     printFlag = FALSE, seed = 4476)
```

Now, we'll fit the pooled kitchen sink regression model to these imputed data sets and pool them.

```
model.empbmi.mi <- with(emp_bmi_mi, lm(bmi ~ age + gender + employed + married +
                                         alcohol + education))
round(summary(pool(model.empbmi.mi))), 3)
```

	est	se	t	df	Pr(> t )	lo 95	hi 95	nmis	fmi
(Intercept)	26.130	0.878	29.772	985	0.000	24.407	27.852	NA	0.004
age	-0.042	0.011	-3.983	985	0.000	-0.063	-0.021	1	0.004
gender2	0.299	0.202	1.482	986	0.139	-0.097	0.695	NA	0.003
employed	-0.465	0.190	-2.442	987	0.015	-0.838	-0.091	1	0.002
married	0.097	0.212	0.456	987	0.649	-0.319	0.512	1	0.002
alcohol2	0.259	0.402	0.645	987	0.519	-0.529	1.048	NA	0.002
alcohol3	0.143	0.403	0.356	987	0.722	-0.647	0.933	NA	0.002
education2	-0.272	0.239	-1.138	983	0.255	-0.741	0.197	NA	0.006
education3	-0.500	0.221	-2.265	982	0.024	-0.933	-0.067	NA	0.007
education4	-0.797	0.309	-2.580	983	0.010	-1.403	-0.191	NA	0.006
lambda									
(Intercept)	0.002								
age	0.002								
gender2	0.001								
employed	0.000								
married	0.000								
alcohol2	0.000								
alcohol3	0.000								
education2	0.004								
education3	0.005								
education4	0.004								

Note that in summarizing these pooled results, R does some strange things:

- it relabels the indicator variables as gender2 rather than gendermale so you need to know the level order for each variable.
- it leaves as NA the number of missing values imputed for every categorical variable it recognizes as a factor.



# Chapter 46

## Species Found on the Galapagos Islands

### 46.1 A Little Background

The `gala` data describe features of the 30 Galapagos Islands.

The Galapagos Islands are found about 900 km west of South America: specifically the continental part of Ecuador. The Islands form a province of Ecuador and serve as a national park and marine reserve. They are noted for their vast numbers of unique (or endemic) species and were studied by Charles Darwin during the voyage of the Beagle. I didn't know most of this, but it's on Wikipedia, so I'll assume it's all true until someone sets me straight.

#### 46.1.1 Sources

The data were initially presented by Johnson M and Raven P (1973) Species number and endemism: the Galapagos Archipelago revisited. *Science* 179: 893-895 and also appear in several regression texts, including my source: Faraway (2015). Note that Faraway filled in some missing data to simplify things a bit. A similar version of the data is available as part of the `faraway` library in R, but I encourage you to use the version I supply on our web site.

#### 46.1.2 Variables in the `gala` data frame

- **id** = island identification code
- **island** = island name
- **species** = our outcome, the number of species found on the island
- **area** = the area of the island, in square kilometers
- **elevation** = the highest elevation of the island, in meters
- **nearest** = the distance from the nearest island, in kilometers
- **scruz** = the distance from Santa Cruz Island, in kilometers. Santa Cruz is the home to the largest human population in the Islands, and to the town of Puerto Ayora.
- **adjacent** = the area of the adjacent island, in square kilometers

`gala`

```
# A tibble: 30 x 8
  id      island species   area elevation nearest scruz adjacent
  <dbl>    <chr>   <dbl>   <dbl>     <dbl>    <dbl>   <dbl>    <dbl>
```

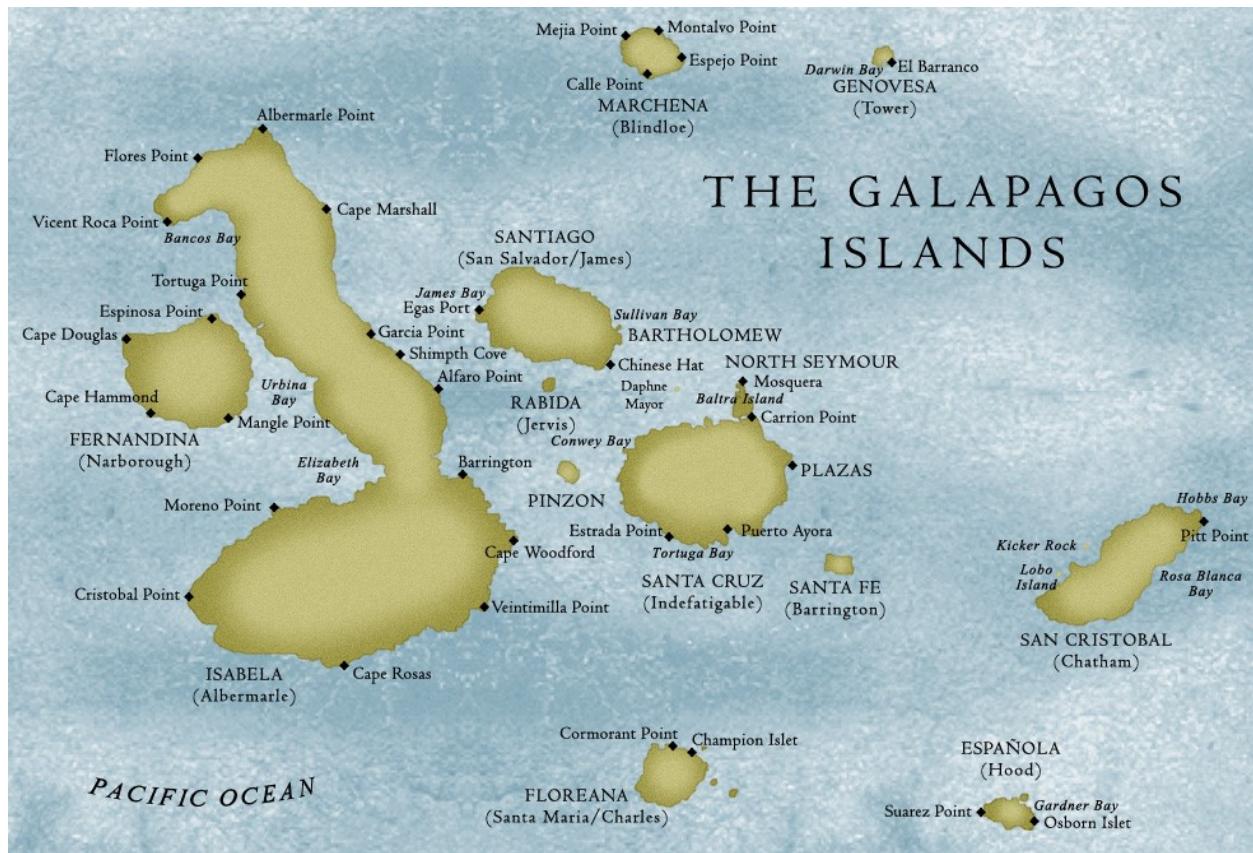


Figure 46.1: galapic.jpg

```

<int>      <fctr>    <int> <dbl>      <int> <dbl> <dbl> <dbl>
1     1       Baltra     58 25.09      346    0.6   0.6   1.84
2     2       Bartolome   31 1.24      109    0.6  26.3 572.33
3     3       Caldwell    3 0.21      114    2.8  58.7  0.78
4     4       Champion   25 0.10      46    1.9  47.4  0.18
5     5       Coamano    2 0.05      77    1.9  1.9 903.82
6     6 Daphne.Major  18 0.34      119    8.0   8.0   1.84
7     7 Daphne.Minor  24 0.08      93    6.0  12.0  0.34
8     8       Darwin    10 2.33      168   34.1 290.2  2.85
9     9       Eden      8 0.03      71    0.4   0.4 17.95
10    10 Enderby     2 0.18      112    2.6  50.2  0.10
# ... with 20 more rows

```

```
Hmisc::describe(gala) # check for missing and inexplicable values
```

```
gala
```

```
8 Variables      30 Observations
```

---

```
id
```

n	missing	distinct	Info	Mean	Gmd	.05	.10
30	0	30	1	15.5	10.33	2.45	3.90
.25	.50	.75	.90	.95			
8.25	15.50	22.75	27.10	28.55			

```
lowest : 1 2 3 4 5, highest: 26 27 28 29 30
```

---

```
island
```

n	missing	distinct
30	0	30

```
lowest : Baltra      Bartolome    Caldwell    Champion    Coamano
highest: SantaFe    SantaMaria  Seymour     Tortuga     Wolf
```

---

```
species
```

n	missing	distinct	Info	Mean	Gmd	.05	.10
30	0	27	0.999	85.23	109.5	2.0	2.9
.25	.50	.75	.90	.95			
13.0	42.0	96.0	280.5	319.1			

```
lowest : 2 3 5 8 10, highest: 237 280 285 347 444
```

---

```
area
```

n	missing	distinct	Info	Mean	Gmd	.05	.10
30	0	29	1	261.7	478.6	0.0390	0.0770
.25	.50	.75	.90	.95			
0.2575	2.5900	59.2375	578.5460	782.6215			

Value	0	20	30	60	130	170	550	570	630	900
Frequency	17	3	1	2	1	1	1	1	1	1
Proportion	0.567	0.100	0.033	0.067	0.033	0.033	0.033	0.033	0.033	0.033

```
Value      4670
```

```
Frequency     1
```

```
Proportion 0.033
```

## elevation

n	missing	distinct	Info	Mean	Gmd	.05	.10
30	0	30	1	368	411.1	47.35	68.80
.25	.50	.75	.90	.95			
97.75	192.00	435.25	868.20	1229.40			

lowest : 25 46 49 71 76, highest: 777 864 906 1494 1707

## nearest

n	missing	distinct	Info	Mean	Gmd	.05	.10
30	0	22	0.997	10.06	13.73	0.445	0.590
.25	.50	.75	.90	.95			
0.800	3.050	10.025	34.100	40.205			

lowest : 0.2 0.4 0.5 0.6 0.7, highest: 16.5 29.1 34.1 45.2 47.4

## scruz

n	missing	distinct	Info	Mean	Gmd	.05	.10
30	0	29	1	56.98	65.17	0.49	0.60
.25	.50	.75	.90	.95			
11.02	46.65	81.08	97.73	193.90			

lowest : 0.0 0.4 0.6 1.9 8.0, highest: 93.1 95.3 119.6 254.7 290.2

## adjacent

n	missing	distinct	Info	Mean	Gmd	.05	.10
30	0	21	0.998	261.1	477.8	0.10	0.10
.25	.50	.75	.90	.95			
0.52	2.59	59.24	578.55	782.62			

Value 0 20 30 60 130 570 630 900 4670

Frequency 17 2 2 2 2 2 1 1 1

Proportion 0.567 0.067 0.067 0.067 0.067 0.067 0.033 0.033 0.033

## 46.2 DTDP: A Scatterplot Matrix

After missingness and range checks, the first step in any data analysis problem is to draw the picture. The most useful picture for me in thinking about a regression problem with a reasonably small number of predictors is a scatterplot matrix.

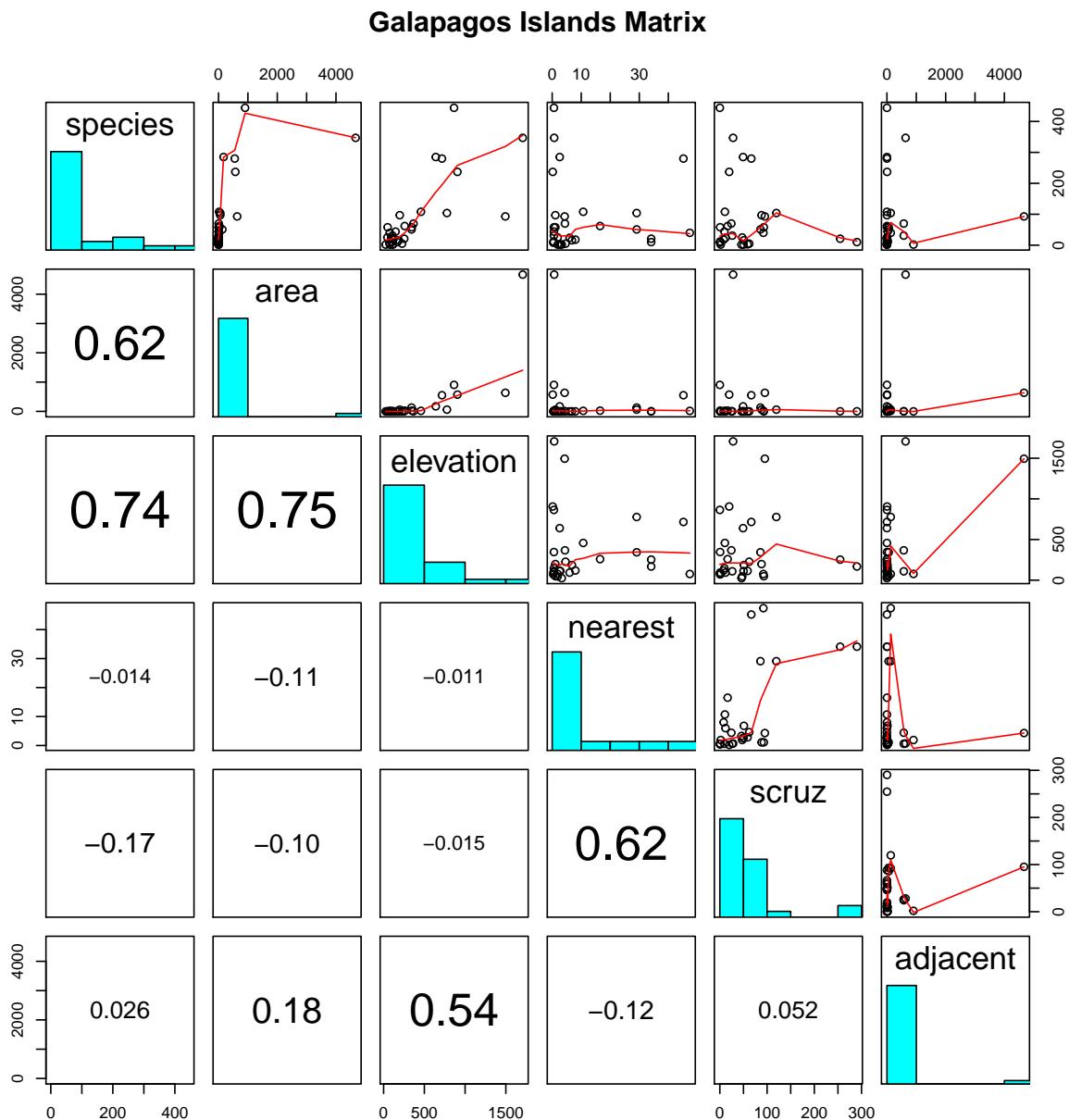
Our outcome, that we are predicting here is the number of species.

We'll use five predictors:

- area
- elevation
- nearest
- scruz
- adjacent.

```
pairs (~ species + area + elevation + nearest + scruz + adjacent,
      data=gala, main="Galapagos Islands Matrix",
```

```
upper.panel = panel.smooth,
diag.panel = panel.hist,
lower.panel = panel.cor)
```



### 46.2.1 Questions about the Scatterplot Matrix

1. What are we looking for in the scatterplots in the top row?
2. What can we learn from the Pearson correlations in the left column?
3. How do the histograms help increase our understanding of the data?
4. What about the scatterplots that are not in the top row?
5. What can we learn from the Pearson correlations that compare predictors?

## 46.3 Fitting A “Kitchen Sink” Linear Regression model

Next, we’ll fit a multiple linear regression model to predict the number of species based on the five predictors included in the `gala` data frame (and scatterplot matrix above.) We use the `lm` command to fit the linear model, and use what is called Wilkinson-Rogers notation to specify the model.

```
model1 <- lm(species ~ area + elevation + nearest + scruz +
               adjacent, data=gala)
summary(model1)
```

Call:  
`lm(formula = species ~ area + elevation + nearest + scruz + adjacent,`  
`data = gala)`

Residuals:

Min	1Q	Median	3Q	Max
-111.68	-34.90	-7.86	33.46	182.58

Coefficients:

	Estimate	Std. Error	t value	Pr(> t )
(Intercept)	7.06822	19.15420	0.37	0.7154
area	-0.02394	0.02242	-1.07	0.2963
elevation	0.31946	0.05366	5.95	3.8e-06 ***
nearest	0.00914	1.05414	0.01	0.9932
scruz	-0.24052	0.21540	-1.12	0.2752
adjacent	-0.07480	0.01770	-4.23	0.0003 ***

Signif. codes: 0 '\*\*\*' 0.001 '\*\*' 0.01 '\*' 0.05 '.' 0.1 ' ' 1

Residual standard error: 61 on 24 degrees of freedom  
Multiple R-squared: 0.766, Adjusted R-squared: 0.717  
F-statistic: 15.7 on 5 and 24 DF, p-value: 6.84e-07

### 46.3.1 Questions about the Kitchen Sink Model Summaries

What conclusions can we draw from the `summary` output for this model? Specifically ...

6. What is being predicted? What is the prediction equation?
7. How do we interpret the `elevation` estimate of 0.32?
8. How do we interpret the `area` estimate of -0.02?
9. How do we interpret the intercept estimate of 7.07?
10. Overall, does the model add statistically significant predictive value over the simplest possible model, using the intercept term alone?
11. What proportion of the variation in `species` counts does this model account for?
12. What does the residual standard error mean in this context?
13. What can we learn from the standard errors in the coefficient output?
14. What can we learn from the `t` values and `Pr(>|t|)` values in the coefficient output?
15. How should we interpret the meaning of the `Adjusted R-squared` value?

## 46.4 Finding Confidence Intervals for our Coefficient Estimates

```
confint(model1, level = 0.95)
```

	2.5 %	97.5 %
(Intercept)	-32.4641	46.6005
area	-0.0702	0.0223
elevation	0.2087	0.4302
nearest	-2.1665	2.1848
scruz	-0.6851	0.2040
adjacent	-0.1113	-0.0383

### 46.4.1 Questions about the Confidence Intervals

16. What can we learn from the provided confidence interval for `elevation`?
17. How do the confidence interval results here compare to the t tests in the `summary` output?

## 46.5 Measuring Collinearity - the Variance Inflation Factor

The **variance inflation factor** (abbreviated VIF) can be used to quantify the impact of multicollinearity in a linear regression model.

The VIF is sometimes interpreted by taking its square root, and then interpreting the result as telling you how much larger the standard error for that coefficient is, as compared to what it would be if that variable were uncorrelated with the other predictors.

In R, the `vif` function from the `car` library, when applied to a linear regression model, specifies the variance inflation factors for each of the model's coefficients, as follows.

```
vif(model1)
```

area	elevation	nearest	scruz	adjacent
2.93	3.99	1.77	1.68	1.83

So, for instance, the VIF of 3.99 for `elevation` implies that the standard error of the elevation coefficient is approximately 2 times larger than it would be if elevation was uncorrelated with the other predictors.

I will look closely at any VIF value that is greater than 5, although some people use a cutoff of 10.

- Another collinearity measure called tolerance is simply  $1/VIF$ .
- For example, the tolerance for `elevation` would be 0.25, and the cutoff for a potentially problematic tolerance is either 0.2 or lower, or 0.1 or lower.

To calculate the VIF for a predictor  $x_1$ , use all of the other predictors to predict  $x_1$  and find the multiple R<sup>2</sup> value.

- VIF for  $x_1 = 1 / (1 - R_{x_1|others}^2)$ , and tolerance =  $(1 - R_{x_1|others}^2)$ .

## 46.6 Global (F) Testing of Overall Significance

Our Galapagos Islands species count regression model (called `model1`) predicts the count of an island's species using `area`, `elevation`, `nearest`, `scruz` and `adjacent`.

```
nullmodel <- lm(species ~ 1, data=gala)
summary(nullmodel)

Call:
lm(formula = species ~ 1, data = gala)

Residuals:
    Min      1Q  Median      3Q     Max 
 -83.2   -72.2   -43.2    10.8   358.8 

Coefficients:
            Estimate Std. Error t value Pr(>|t|)    
(Intercept)  85.2       20.9     4.07  0.00033 ***
---
Signif. codes:  0 '***' 0.001 '**' 0.01 '*' 0.05 '.' 0.1 ' ' 1

Residual standard error: 115 on 29 degrees of freedom
anova(model1, nullmodel)
```

#### Analysis of Variance Table

```
Model 1: species ~ area + elevation + nearest + scruz + adjacent
Model 2: species ~ 1
Res.Df   RSS Df Sum of Sq   F  Pr(>F)    
1     24  89231
2     29 381081 -5   -291850 15.7 6.8e-07 ***
---
Signif. codes:  0 '***' 0.001 '**' 0.01 '*' 0.05 '.' 0.1 ' ' 1
```

### 46.6.1 Questions about the Global Test via ANOVA

18. How do we interpret the null model fit above?
19. What are the hypotheses being tested by this ANOVA output?
20. What conclusions can we draw from the ANOVA output presented here?
21. Where do we find information regarding the result for the previous question in the summary output for the linear model?
22. How would we set up an ANOVA model to test whether the “kitchen sink” model’s predictive value would be significantly impacted by removing the `adjacent` predictor from the model?
23. Where do we find information regarding these result for the previous question in the `summary` output for the linear model?
24. How would we set an ANOVA model to test whether a model with `area` only would be a significant improvement over the null model?

### 46.7 Sequential Testing in a Regression Model with ANOVA

```
anova(model1)

Analysis of Variance Table

Response: species
```

```

      Df Sum Sq Mean Sq F value    Pr(>F)
area      1 145470  145470   39.13 1.8e-06 ***
elevation 1  65664   65664   17.66 0.00032 ***
nearest   1     29     29   0.01 0.93007
scruz     1 14280   14280   3.84 0.06173 .
adjacent  1  66406   66406   17.86 0.00030 ***
Residuals 24  89231   3718
---
Signif. codes:  0 '***' 0.001 '**' 0.01 '*' 0.05 '.' 0.1 ' ' 1

```

### 46.7.1 Questions about Sequential Testing and ANOVA

25. What conclusions can we draw from the `area` row in the output above?
26. What conclusions can we draw from the `elevation` row?
27. Does `nearest` add statistically significant predictive value to the model including `area` and `elevation`, but none of the other predictors?
28. Does `adjacent` add significant predictive value as last predictor into the model?
29. Where else in the regression output can we find the answer to the previous question?
30. How does the mean square of the residuals (3718) relate to the residual standard error?
31. What percentage of the variation in the species counts is accounted for by `area` alone?
32. What percentage of the variation explained by the kitchen sink model would also be accounted for in a two-predictor regression model including `area` and `elevation` alone?
33. How could we use the original linear model output to whether a model using the four predictors that appear most promising here would be statistically significantly worse than the full model with all five predictors?
34. What does the following output do differently than the output above, and why is that potentially useful here? Why is the p value for `scruz` so different?

```
anova(lm(species ~ area + elevation + adjacent + scruz + nearest, data=gala))
```

Analysis of Variance Table

```

Response: species
      Df Sum Sq Mean Sq F value    Pr(>F)
area      1 145470  145470   39.13 1.8e-06 ***
elevation 1  65664   65664   17.66 0.00032 ***
adjacent  1  73171   73171   19.68 0.00017 ***
scruz     1   7544    7544   2.03 0.16719
nearest   1     0     0   0.00 0.99315
Residuals 24  89231   3718
---
Signif. codes:  0 '***' 0.001 '**' 0.01 '*' 0.05 '.' 0.1 ' ' 1

```

35. Consider the ANOVA below, run on a new model with `elevation` after `adjacent`. What happens? Why?

```
anova(lm(species ~ area + adjacent + elevation + scruz + nearest, data=gala))
```

Analysis of Variance Table

```

Response: species
      Df Sum Sq Mean Sq F value    Pr(>F)
area      1 145470  145470   39.13 1.8e-06 ***
adjacent  1   2850    2850   0.77   0.39
elevation 1 135985  135985  36.58 3.0e-06 ***

```

```

scruz      1    7544    7544    2.03    0.17
nearest    1      0      0    0.00    0.99
Residuals  24   89231   3718
---
Signif. codes:  0 '***' 0.001 '**' 0.01 '*' 0.05 '.' 0.1 ' ' 1

```

## 46.8 An ANOVA table for the Model as a Whole

It's probably also worthwhile to compute a completed ANOVA table for the model as a whole. All elements are in the ANOVA tables above, or the model summary.

Group	DF	SS	MS	F	P
Regression	5	291849	58369.8	15.7	6.838e-07
Residuals	24	89231	3718.0		
Total	29	381080			

36. How did I determine the Mean Square for the Regression model?
37. What conclusions can we draw from this ANOVA table?

## 46.9 Assumption Checking for our Galápagos Islands models

Remember that the key assumptions of multiple linear regression are:

- [Linearity] We have also assumed that the structural part of the model is correctly specified (we've included all the predictors that need to be in the model, and expressed them in the most appropriate manner, and we've left out any predictors that don't need to be in the model.)
- [Normality] The regression makes errors that come from a Normal distribution
- [Homoscedasticity = Constant Variance] The regression makes errors that come from a distribution with constant variance at all predictor levels.
- [Independence] The regression errors are independent of each other.

In addition, we need to realize that sometimes a few observations may be particularly problematic. For instance:

1. An observation may simply not fit the model well (i.e. it creates a large residual)
2. An observation may have high leverage over the fit of the model (this happens with observations that have an unusual combination of predictor values, in particular)
3. An observation may actually have high influence on the model (in the sense that whether they are included or excluded has a large impact on the model's fit, and the value of its parameter estimates.)
4. Or any combination of high residual, leverage and influence may occur.

So it is important to check the assumptions that we can with the data we have. Our most important tools are plots and other summaries of residuals, and what are called influence statistics.

## 46.10 My First Plot: Studentized Residuals vs. Fitted Values

The first diagnostic plot I usually draw for a multiple regression is a scatterplot of the model's **studentized** residuals<sup>1</sup> (on the vertical axis) vs. the model's fitted values (on the horizontal.) This plot can be used to assess potential non-linearity, non-constant variance, and non-Normality in the residuals.

---

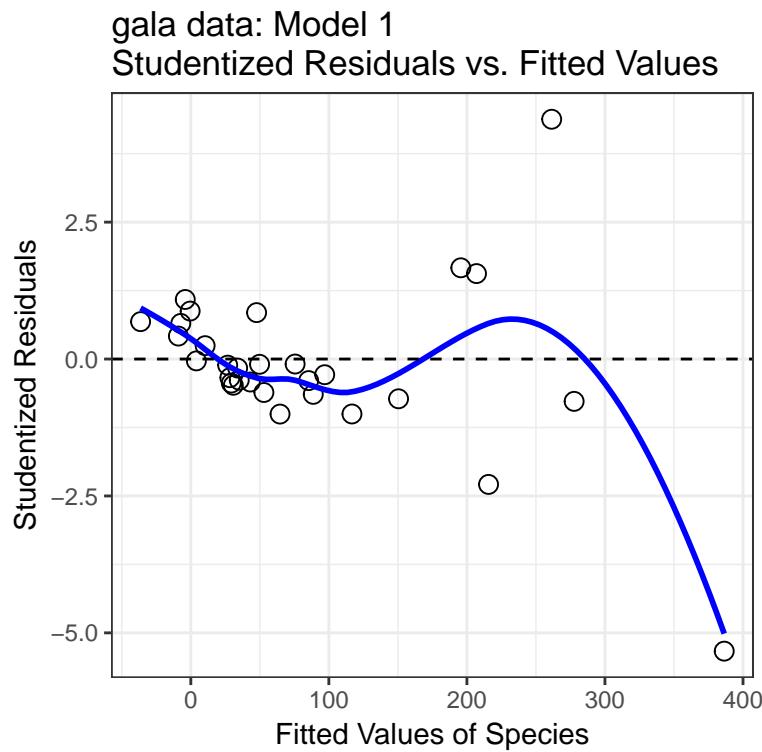
<sup>1</sup>More on studentized and standardized residuals later. For now, think of them like z scores.

```

gala$stures <- rstudent(model1); gala$fits <- fitted(model1)
ggplot(gala, aes(x = fits, y = stures)) +
  theme_bw() + geom_point(size = 3, shape = 1) +
  geom_smooth(col = "blue", se = FALSE, weight = 0.5) +
  geom_hline(aes(yintercept = 0), linetype = "dashed") +
  labs(x = "Fitted Values of Species",
       y = "Studentized Residuals",
       title = "gala data: Model 1\nStudentized Residuals vs. Fitted Values")

```

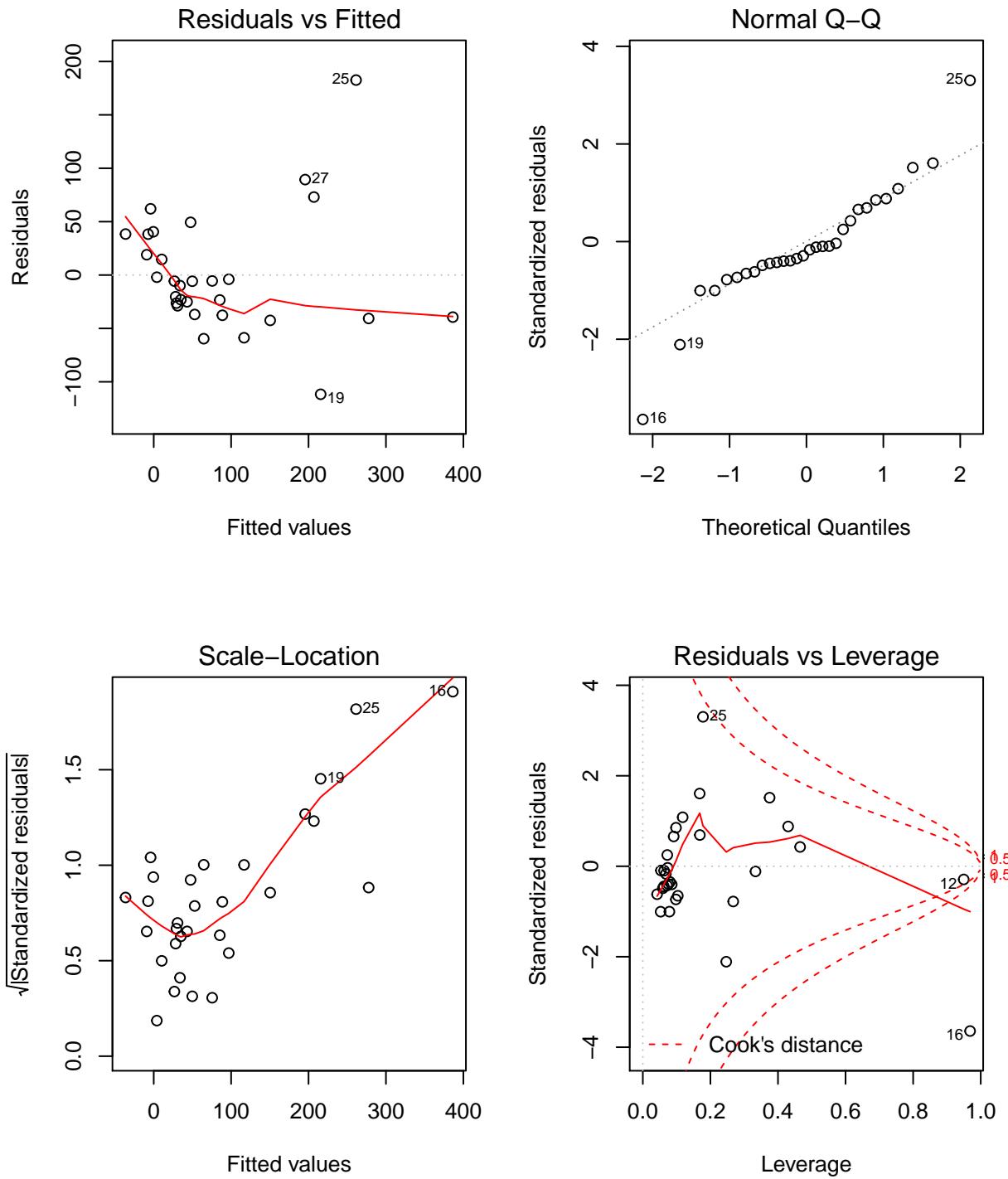
`geom\_smooth()` using method = 'loess'



#### 46.10.1 Questions about Studentized Residuals vs. Fitted Values

38. Consider the point at bottom right. What can you infer about this observation?
39. Why did I include the dotted horizontal line at Studentized Residual = 0?
40. What is the purpose of the thin blue line?
41. What does this plot suggest about the potential for outliers in the residuals?

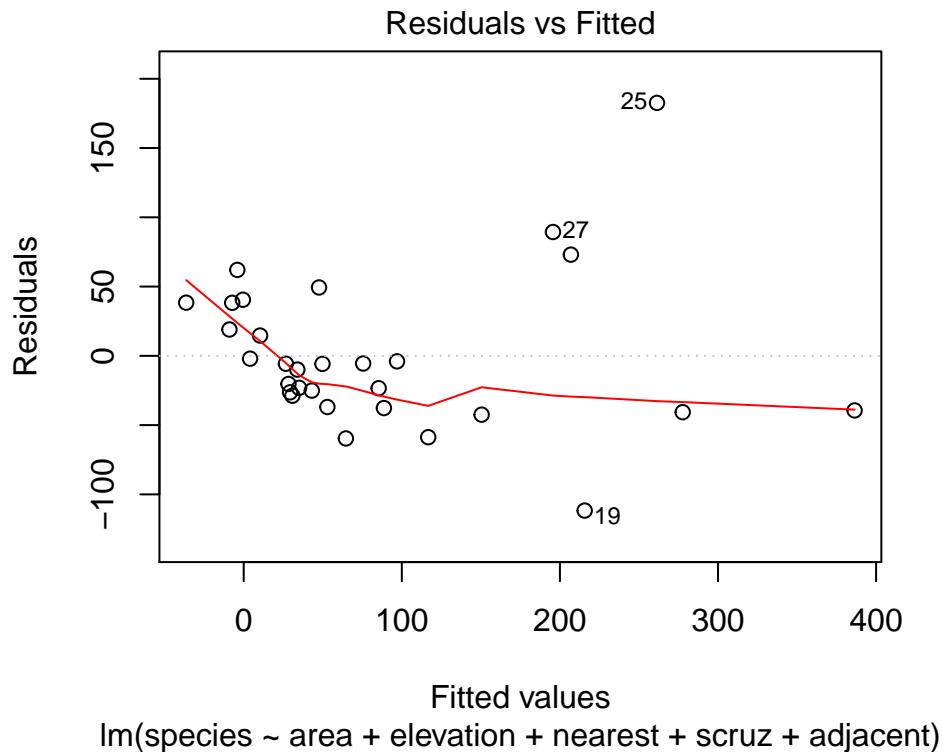
### 46.11 Automatic Regression Diagnostics for Model 1



## 46.12 Model 1: Diagnostic Plot 1

As we've seen, the first of R's automated diagnostic plots for a linear model is a plot of the residuals vs. the fitted values.

```
plot(model1, which=1)
```



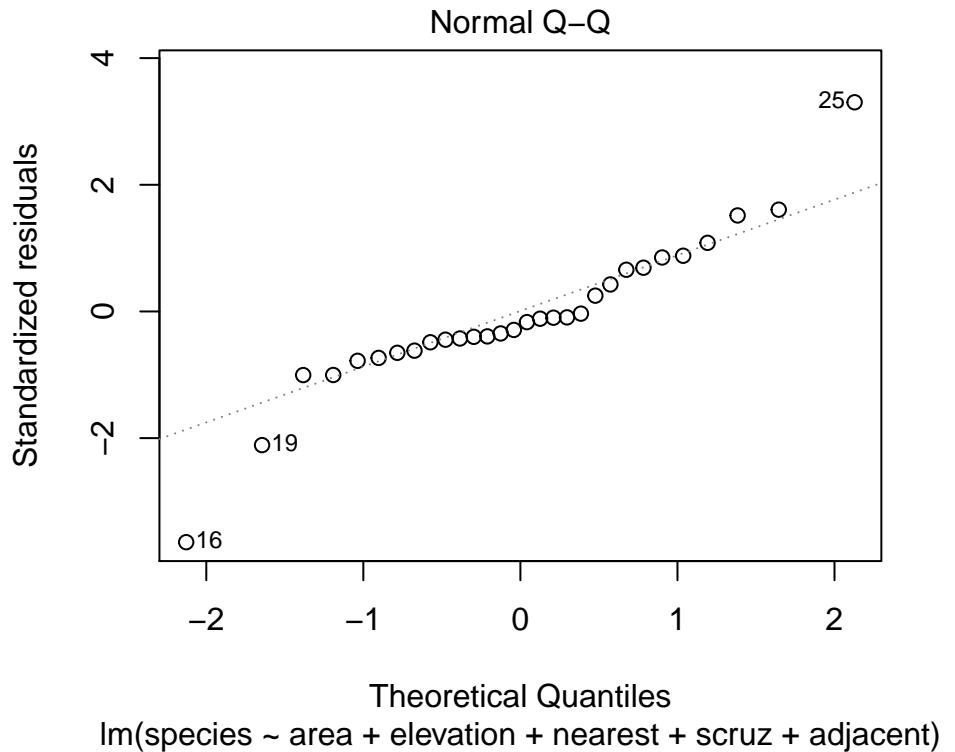
### 46.12.1 Questions about Diagnostic Plot 1: Residuals vs. Fitted Values

42. What type of regression residuals is R plotting here?
43. Which points are identified by numbers here?
44. Why did R include the gray dotted line at Residual = 0?
45. What is the purpose of the thin red line?
46. What can you tell about the potential for outliers in the model 1 residuals from the plot?
47. What are we looking for in this plot that would let us conclude there were no important assumption violations implied by it? Which assumptions can we assess with it?
48. What would we do if we saw a violation of assumptions in this plot?
49. What are the key differences between this plot and the one I showed earlier?

## 46.13 Diagnostic Plot 2: Assessing Normality

The second diagnostic plot prepared by R for any linear model using the plot command is a Normal Q-Q plot of the standardized residuals from the model.

```
plot(model1, which=2)
```



#### 46.13.1 Questions about Diagnostic Plot 2: Normal Plot of Standardized Residuals

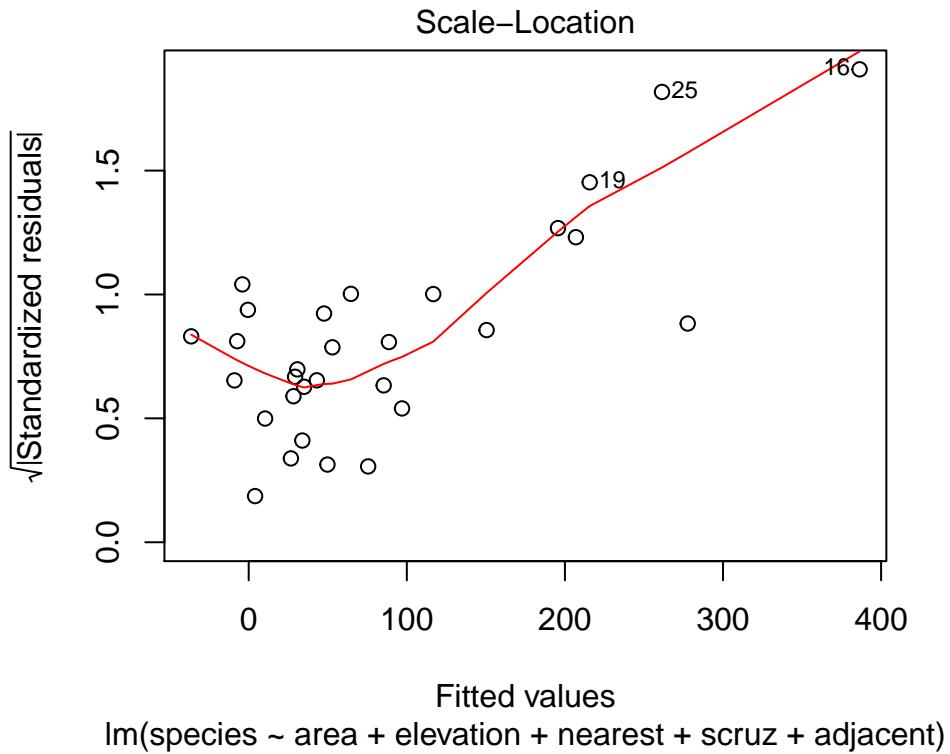
50. Which points are being identified here by number?
51. Which assumption(s) of multiple regression does this plot help us check?
52. What are we looking for in this plot that would let us conclude there were no important assumption violations implied by it?
53. What would we do if we saw a violation of assumptions in this plot?

We could also look at studentized residuals, or we could apply a more complete set of plots and other assessments of normality. Usually, I don't.

#### 46.14 Diagnostic Plot 3: Assessing Constant Variance

The third diagnostic plot prepared by R for any linear model using the `plot` command shows the square root of the model's standardized residuals vs. its fitted values. R calls this a **scale-location plot**.

```
plot(model1, which=3)
```



#### 46.14.1 Questions about Diagnostic Plot 3: Scale-Location Plot

54. Which points are being identified here by number?
55. Which assumption(s) of multiple regression does this plot help us check?
56. What is the role of the thin red line in this plot?
57. What are we looking for in this plot that would let us conclude there were no important assumption violations implied by it?
58. What would we do if we saw a violation of assumptions in this plot?

## 46.15 Obtaining Fitted Values and Residuals from a Model

Remember that we can use the `fitted` function applied to a model to find the predictions made by the regression model for each of the observations used to create the model.

```
round(fitted(model1), 2)
```

1	2	3	4	5	6	7	8	9	10
116.73	-7.27	29.33	10.36	-36.38	43.09	33.92	-9.02	28.31	30.79
11	12	13	14	15	16	17	18	19	20
47.66	96.99	-4.03	64.63	-0.50	386.40	88.69	4.04	215.68	150.48
21	22	23	24	25	26	27	28	29	30
35.08	75.55	206.95	277.68	261.42	85.38	195.62	49.81	52.94	26.70

```
gala[1,]
```

```
# A tibble: 1 x 10
  id island species area elevation nearest scruz adjacent stures fits
  <int> <fctr>    <int> <dbl>      <int>   <dbl> <dbl>     <dbl> <dbl> <dbl>
1     1 Baltra      58  25.1       346     0.6   0.6     1.84    -1    117
```

### 46.15.1 Questions about Fitted Values

59. Verify that the first fitted value [116.73] is in fact what you get for Baltra (observation 1) when you apply the regression equation:

```
species = 7.07 - 0.02 area + 0.32 elevation
          + 0.009 nearest - 0.24 scruz - 0.07 adjacent
```

We can compare these predictions to the actual observed counts of the number of species on each island. Subtracting the fitted values from the observed values gives us the residuals, as does the `resid` function.

```
round(resid(model1),2)
```

1	2	3	4	5	6	7	8	9
-58.73	38.27	-26.33	14.64	38.38	-25.09	-9.92	19.02	-20.31
10	11	12	13	14	15	16	17	18
-28.79	49.34	-3.99	62.03	-59.63	40.50	-39.40	-37.69	-2.04
19	20	21	22	23	24	25	26	27
-111.68	-42.48	-23.08	-5.55	73.05	-40.68	182.58	-23.38	89.38
28	29	30						
-5.81	-36.94	-5.70						

### 46.15.2 Questions about Residuals

60. What does a positive residual indicate?  
 61. What does a negative residual indicate?  
 62. The standard deviation of the full set of 30 residuals turns out to be 55.47. How does this compare to the residual standard error?  
 63. The command below identifies Santa Cruz. What does it indicate about Santa Cruz, specifically?

```
gala$island[which.max(resid(model1))]
```

```
[1] SantaCruz
30 Levels: Baltra Bartolome Caldwell Champion Coamano ... Wolf
```

64. From the results below, what is the `model1` residual for Santa Cruz? What does this imply about the `species` prediction made by Model 1 for Santa Cruz?

```
which.max(resid(model1))
```

```
25
25
```

```
round(resid(model1),2)
```

1	2	3	4	5	6	7	8	9
-58.73	38.27	-26.33	14.64	38.38	-25.09	-9.92	19.02	-20.31
10	11	12	13	14	15	16	17	18
-28.79	49.34	-3.99	62.03	-59.63	40.50	-39.40	-37.69	-2.04
19	20	21	22	23	24	25	26	27

```

-111.68 -42.48 -23.08 -5.55 73.05 -40.68 182.58 -23.38 89.38
 28      29      30
-5.81 -36.94 -5.70
gala[which.max(resid(model1)),]

```

```

# A tibble: 1 x 10
  id    island species area elevation nearest scruz adjacent stures
  <int>   <fctr>   <int> <dbl>     <int>   <dbl>   <dbl>     <dbl>   <dbl>
1 25 SantaCruz     444    904     864     0.6     0     0.52    4.38
# ... with 1 more variables: fits <dbl>

```

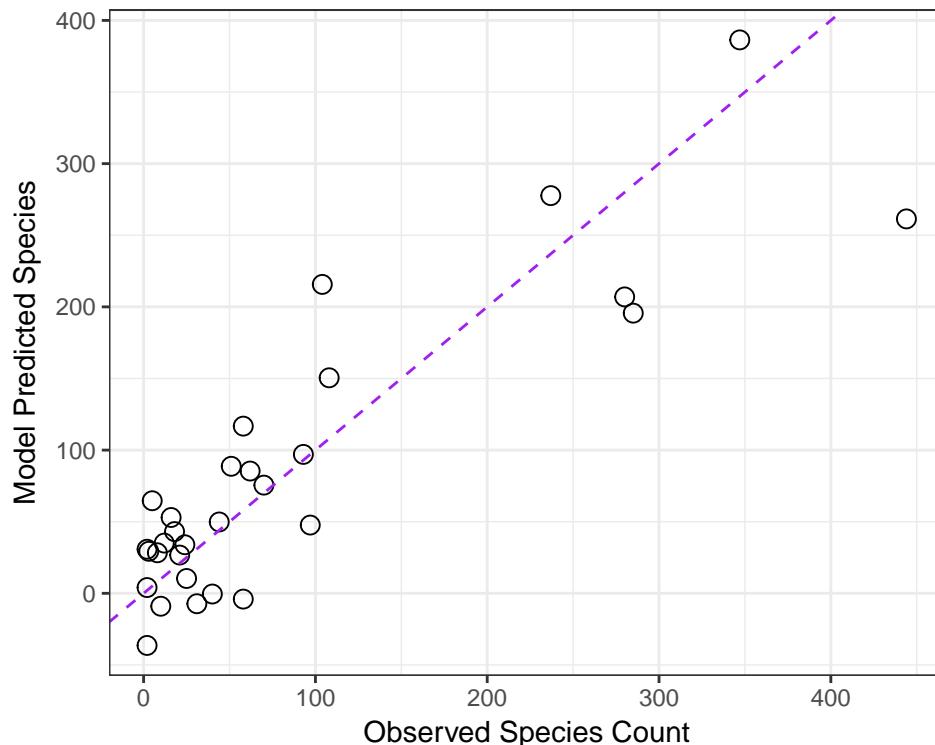
## 46.16 Relationship between Fitted and Observed Values

We've already seen that the `fitted` command can produce predicted values for each observations used to develop a regression model, and that the `resid` command can produce the residuals (observed - predicted) for those same observations. Returning to our original `model1`, let's compare the fitted values (stored earlier in `fits`) to the observed values.

```

ggplot(gala, aes(x = species, y = fits)) +
  geom_point(size = 3, shape = 1) + theme_bw() +
  geom_abline(intercept = 0, slope = 1, col = "purple", linetype = "dashed") +
  labs(x = "Observed Species Count", y = "Model Predicted Species")

```



### 46.16.1 Questions about Fitted and Observed Values

65. Why did I draw the dotted purple line with y-intercept 0 and slope 1? Why is that particular line of interest?

66. If a point on this plot is in the top left here, above the dotted line, what does that mean?
67. If a point is below the dotted line here, what does that mean?
68. How does this plot display the size of an observation's residual?

## 46.17 Standardizing Residuals

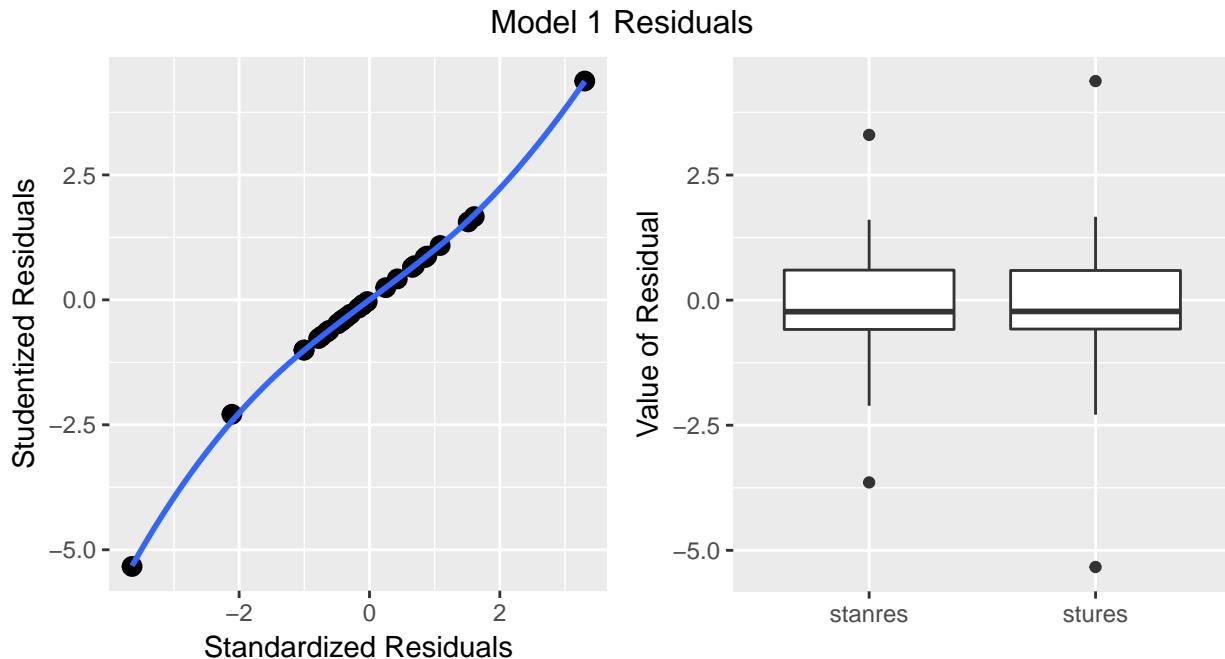
We've already seen that the raw residuals from a regression model can be obtained using the `resid` function. Residuals are defined to have mean 0. This is one of the requirements of the least squares procedure for estimating a linear model, and their true standard deviation is effectively estimated using the residual standard error.

There are two additional types of residuals for us to be aware of: standardized residuals, and studentized (sometimes called externally standardized, or jackknife) residuals. Each approach standardizes the residuals by dividing them by a standard deviation estimate, so the resulting residuals should have mean 0 and standard deviation 1 if assumptions hold.

- **Standardized** residuals are the original (raw) residuals, scaled by a standard deviation estimate developed using the entire data set.
- **Studentized** residuals are the original (raw) residuals, scaled by a standard deviation estimate developed using the entire data set EXCEPT for this particular observation.

The `rstandard` function, when applied to a linear regression model, will generate the standardized residuals, while `rstudent` generates the model's studentized residuals.

```
`geom_smooth()` using method = 'loess'
```



### 46.17.1 Questions about Standardized and Studentized Residuals

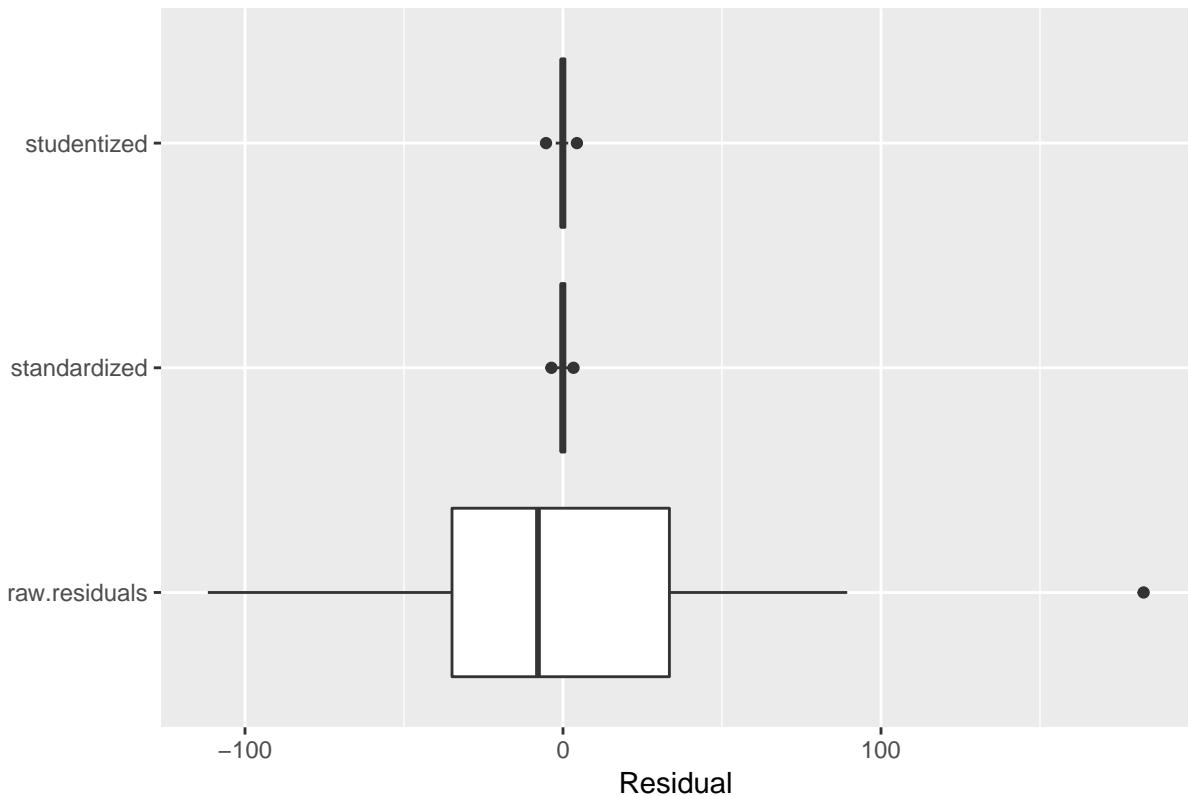
69. From the plots above, what conclusions can you draw about the two methods of standardizing residuals as they apply in the case of our model1?

## 46.18 Three Types of Residuals

```
gala.res <- data.frame(raw.residuals = resid(model1),
                        standardized = rstandard(model1),
                        studentized = rstudent(model1)) %>%tbl_df

gala.res_long <- gather(gala.res, key = "type", value = "res")
ggplot(gala.res_long, aes(x = type, y = res)) +
  geom_boxplot() +
  coord_flip() +
  labs(x = "", y = "Residual", title = "3 Types of Residuals for Model 1")
```

3 Types of Residuals for Model 1

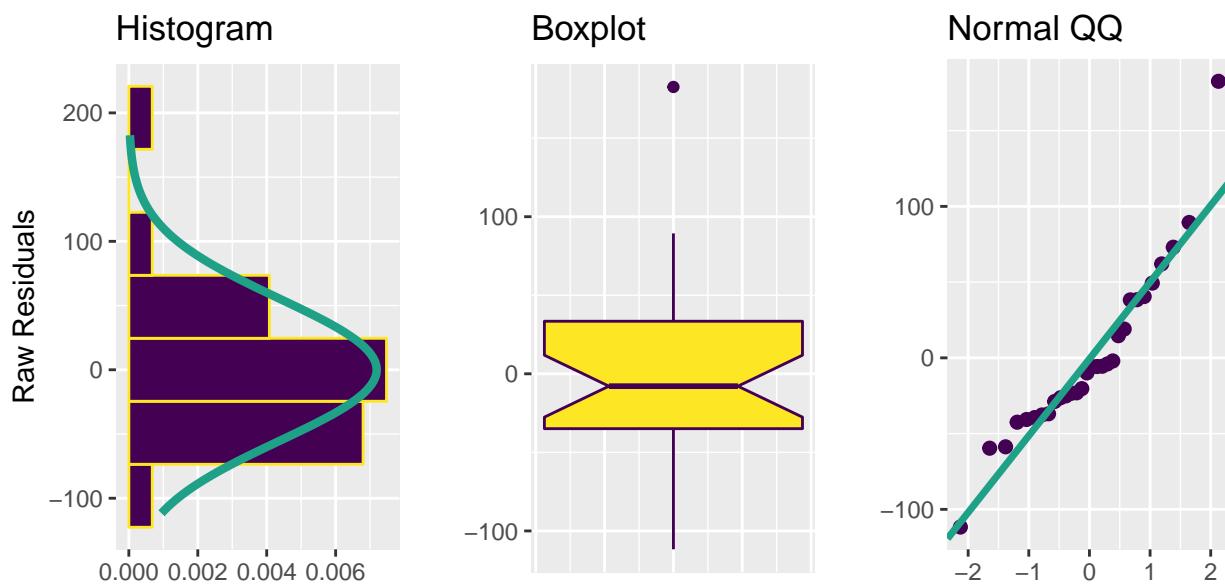


### 46.18.1 Questions about Three Types of Residuals

70. Consider the three types of residuals, shown above. Can you specify a reason why looking at the raw residuals might be helpful in this case?
71. Why might (either of the two approaches to) standardizing be useful?
72. Does there seem to be a substantial problem with Normality in the residuals?
73. How about the Normality of the studentized residuals? Which seems clearer?

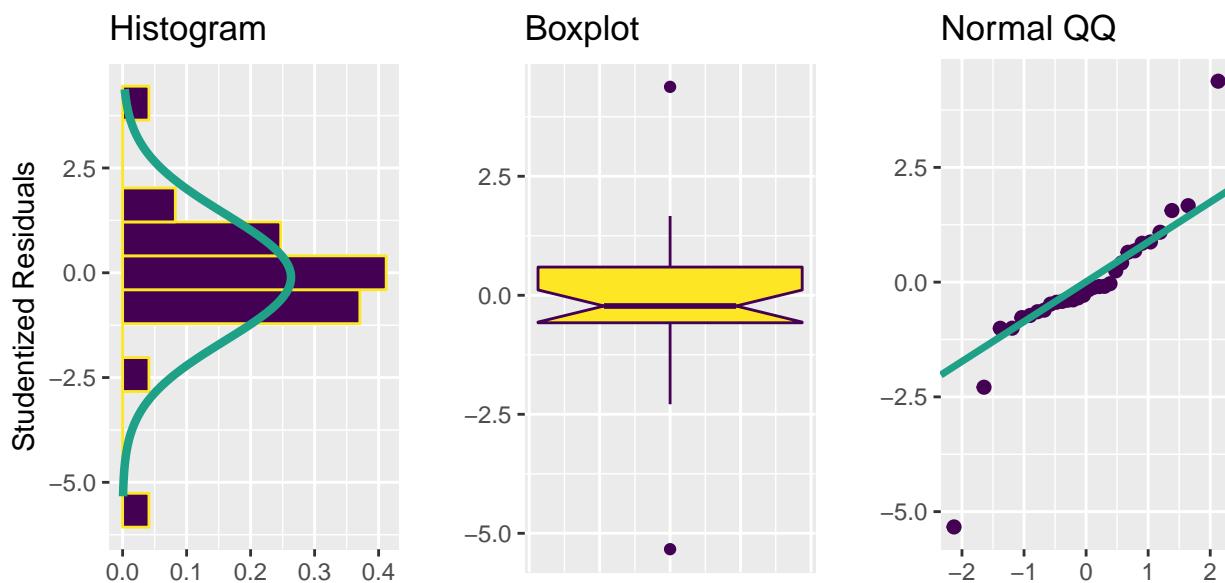
```
eda.1sam(dataframe = gala.res, variable = gala.res$raw.residuals,
          x.title = "Raw Residuals", ov.title = "Model 1: Raw Residuals")
```

## Model 1: Raw Residuals



```
eda.1sam(dataframe = gala.res, variable = gala.res$studentized,
          x.title = "Studentized Residuals",
          ov.title = "Model 1: Studentized Residuals")
```

## Model 1: Studentized Residuals



## Chapter 47

# Influence Measures for Multiple Regression

R can output a series of **influence measures** for a regression model. Let me show you all of the available measures for model 1, but just for three of the data points - #1 (which is not particularly influential) and #12 and #16 (which are).

First, we'll look at the raw data:

```
gala[c(1,12,16),]
```

```
# A tibble: 3 x 11
  id      island species   area elevation nearest scruz adjacent stures
  <int>    <fctr>   <int>   <dbl>     <int>   <dbl>   <dbl>     <dbl>
1     1     Baltra     58   25.1      346     0.6    0.6    1.84 -1.004
2    12 Fernandina    93  634.5     1494     4.3   95.3  4669.32 -0.286
3    16    Isabela   347 4669.3     1707     0.7   28.1   634.49 -5.334
# ... with 2 more variables: fits <dbl>, stanres <dbl>
```

And then, we'll gather the output available in the **influence.measures** function.

```
influence.measures(model1)
```

Here's an edited version of this output...

```
Influence measures of
lm(formula = species ~ area + elevation + nearest + scruz + adjacent,
data = gala) :

  dfb.1_  dfb.area  dfb.elvt dfb.nrst  dfb.scrz  dfb.adjc
1 -0.15064  0.13572 -0.122412  0.07684  0.084786  1.14e-01
12  0.16112  0.16395 -0.122578  0.03093 -0.059059 -8.27e-01
16 -1.18618 -20.87453  4.885852  0.36713 -1.022431 -8.09e-01

  dffit   cov.r   cook.d     hat inf
1  -0.29335  1.0835 1.43e-02  0.0787
12 -1.24249 25.1101 2.68e-01  0.9497  *
16 -29.59041  0.3275 6.81e+01  0.9685  *
```

This output presents dfbetas for each coefficient, followed by dffit statistics, covariance ratios, Cook's distance and leverage values (**hat**) along with an indicator of influence.

We'll consider each of these elements in turn.

## 47.1 DFBETAs

The first part of the influence measures output concerns what are generally called `dfbetas` ...

id	island	dfb.1_	dfb.area	dfb.elvt	dfb.nrst	dfb.scrz	dfb.adjc
1	Baltra	-0.151	0.136	-0.122	0.077	0.085	0.114
12	Fernandina	0.161	0.164	-0.123	0.031	-0.059	-0.827
16	Isabela	-1.186	-20.875	4.886	0.367	-1.022	-0.809

The `dfbetas` look at a standardized difference in the estimate of a coefficient (slope) that will occur if the specified point (here, `island`) is removed from the data set.

- Positive values indicate that deleting the point will yield a smaller coefficient.
- Negative values indicate that deleting the point will yield a larger coefficient.
- If the absolute value of the dfbeta is greater than  $2/\sqrt{n}$ , where  $n$  is the sample size, then the `dfbeta` is considered to be large.

In this case, our cutoff would be  $2/\sqrt{30}$  or 0.365, so that the Isabela `dfbeta` values are all indicative of large influence. Essentially, if we remove Isabela from the data, and refit the model, our regression slopes will change a lot (see below). Fernandina has some influence as well, especially on the `adjacent` coefficient.

Predictor	Coefficient ( $p$ ) all 30 islands	Coefficient ( $p$ ) without Isabela
Intercept	7.07 ( $p = 0.72$ )	22.59 ( $p = 0.11$ )
area	-0.02 ( $p = 0.30$ )	0.30 ( $p < 0.01$ )
elevation	0.32 ( $p < 0.01$ )	0.14 ( $p < 0.01$ )
nearest	0.01 ( $p = 0.99$ )	-0.26 ( $p = 0.73$ )
scruz	-0.24 ( $p = 0.28$ )	-0.09 ( $p = 0.55$ )
adjacent	-0.08 ( $p < 0.01$ )	-0.07 ( $p < 0.01$ )

## 47.2 Other Available Influence Measures

After the `dfbetas`, the `influence.measures` output presents `dffit`, covariance ratios, Cook's distance and leverage values (`hat`) for each observation, along with an indicator of influence.

id	island	dffit	cov.r	cook.d	hat	inf
1	Baltra	-0.29335	1.0835	1.43e-02	0.0787	
12	Fernandina	-1.24249	25.1101	2.68e-01	0.9497	*
16	Isabela	-29.59041	0.3275	6.81e+01	0.9685	*

### 47.2.1 Cook's d or Cook's Distance

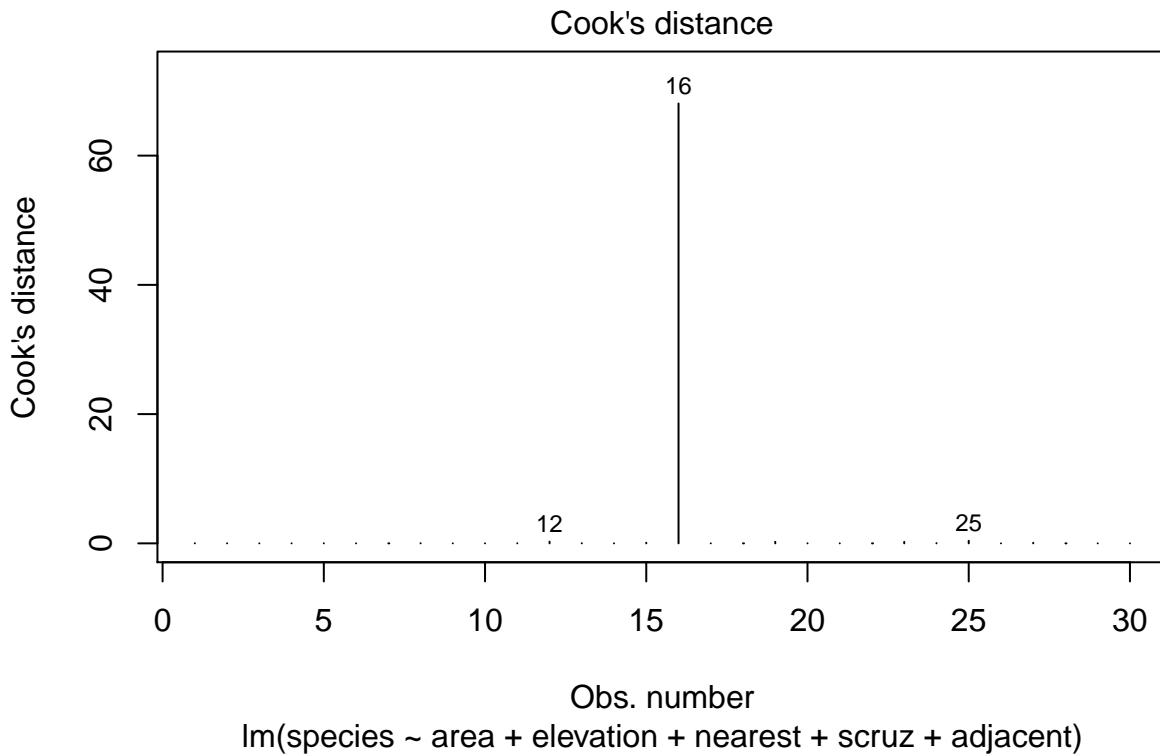
The main measure of influence is Cook's Distance, also called Cook's d. Cook's d provides a summary of the influence of a particular point on all of the regression coefficients. It is a function of the standardized residual and the leverage.

- Cook's distance values greater than 1 are generally indicators of high influence.
- Obviously, Isabela (with a value of Cook's d = 68.1) is a highly influential observation by this measure.

### 47.2.2 Plotting Cook's Distance

As one of its automated regression diagnostic plots, R will produce an index plot of the Cook's distance values. Note the relatively enormous influence for island 16 (Isabela).

```
plot(model1, which = 4)
```



### 47.2.3 DFFITS

A similar measure to Cook's distance is called DFFITS. The DFFITS value describes the influence of the point on the fitted value. It's the number of standard deviations that the fitted value changes if the observation is removed. This is defined as a function of the studentized residual and the leverage.

- If the absolute value of DFFITS is greater than  $2 \sqrt{p/n - p}$ , where  $p$  is the number of predictors (not including the intercept), we deem the observation influential.
- For the `gala` data, we'd consider any point with DFFITS greater than  $2 \times \sqrt{5/(30 - 5)} = 0.894$  to be influential by this standard, since  $n = 30$  and we are estimating  $p = 5$  slopes in our model. This is true of both Fernandina and Isabela.

### 47.2.4 Covariance Ratio

The covariance ratio `cov.r` indicates the role of the observation on the precision of estimation. If `cov.r` is greater than 1, then this observation improves the precision, overall, and if it's less than 1, the observation drops the precision of estimation, and these are the points about which we'll be most concerned.

- As with most of our other influence measures, Isabela appears to be a concern.

### 47.2.5 Leverage

The `hat` value is a measure of leverage. Specifically, this addresses whether or not the point in question is unusual in terms of its combination of predictor values.

- The usual cutoff for a large leverage value is 2.5 times the average leverage across all observations, where the average leverage is equal to  $k/n$ , where  $n$  is the number of observations included in the regression model, and  $k$  is the number of model coefficients (slopes plus intercept).
- In the `gala` example, we'd regard any observation with a hat value larger than  $2.5 \times 6/30 = 0.5$  to have large leverage. This includes Fernandina and Isabela.

### 47.2.6 Indicator of Influence

The little asterisk indicates an observation which is influential according to R's standards for any of these measures. You can take the absence of an asterisk as a clear indication that a point is NOT influential. Points with asterisks may or may not be influential in an important way. In practice, I usually focus on the Cook's distance to make decisions about likely influence, when the results aren't completely clear.

# Chapter 48

## Building Predictions from our models

The `predict` function, when applied to a linear regression model, produces the fitted values, just as the `fitted` function did, and, as we've seen, it can be used to generate *prediction* intervals for a single new observation, or *confidence* intervals for a group of new observations with the same predictor values.

### 48.1 Predictions for a “typical” island

Let us, just for a moment, consider a “typical” island, exemplified by the median value of all the predictors<sup>1</sup>. There's a trick to creating this and dumping it in a vector I will call `x.medians`.

```
x <- model.matrix(model1)
x.medians <- apply(x, 2, function(x) median(x))
x.medians
```

	(Intercept)	area	elevation	nearest	scruz	adjacent
1.00	2.59	192.00	3.05	46.65	2.59	

We want to use the model to predict our outcome (`species`) on the basis of the inputs above: a new island with values of all predictors equal to the median of the existing islands. As before, building an interval forecast around a fitted value requires us to decide whether we are:

- predicting the number of species for one particular island with the specified characteristics (in which case we use something called a prediction interval) or
- predicting the mean number of species across all islands that have the specified characteristics (in which case we use the confidence interval).

```
newdata <- data.frame(t(x.medians))
predict(model1, newdata, interval="prediction", level = 0.95)
```

```
fit lwr upr
1 57 -72.1 186

predict(model1, newdata, interval="confidence", level = 0.95)

fit lwr upr
1 57 28.5 85.4
```

<sup>1</sup>This approach is motivated by @Faraway2015, pp. 52-53.

### 48.1.1 Questions about the Prediction and Confidence Interval Methods

74. What is the 95% prediction interval for this new observation? Does that make sense?
75. Which interval (prediction or confidence) is wider? Does that make sense?
76. Is there an island that has characteristics that match our new medians variable?
77. What happens if we don't specify new data in making a prediction?

## 48.2 Making a Prediction with New Data

78. How does the output below help us to make a prediction with a new data point, or series of them?  
Interpret the resulting intervals.

```
newdata2 <- data.frame(area = 2, elevation = 100, nearest = 3,
                       scruz = 5, adjacent = 1)
predict(model1, newdata2, interval="prediction", level = 0.95)

  fit  lwr upr
1 37.7 -92.5 168

predict(model1, newdata2, interval="confidence", level = 0.95)

  fit  lwr upr
1 37.7  4.39  71
```

# Chapter 49

## Standardizing/Rescaling in Regression Models

### 49.1 Scaling Predictors using Z Scores: Semi-Standardized Coefficients

We know that the interpretation of the coefficients in a regression model is sensitive to the scale of the predictors. We have already seen how to “standardize” each predictor by subtracting its mean and dividing by its standard deviation.

- Each coefficient in this semi-standardized model has the following interpretation: the expected difference in the outcome, comparing units (subjects) that differ by one standard deviation in the variable of interest, but for which all other variables are fixed at their average.
- Remember also that the intercept in such a model shows the mean outcome across all subjects.

Consider a two-variable model, using `area` and `elevation` to predict the number of `species`...

```
model2 <- lm(species ~ area + elevation, data=gala)
summary(model2)
```

Call:  
`lm(formula = species ~ area + elevation, data = gala)`

Residuals:

Min	1Q	Median	3Q	Max
-192.62	-33.53	-19.20	7.54	261.51

Coefficients:

	Estimate	Std. Error	t value	Pr(> t )		
(Intercept)	17.1052	20.9421	0.82	0.4212		
area	0.0188	0.0259	0.72	0.4748		
elevation	0.1717	0.0532	3.23	0.0032 **		
---						
Signif. codes:	0 '***'	0.001 '**'	0.01 '*'	0.05 '.'	0.1 ''	1

Residual standard error: 79.3 on 27 degrees of freedom  
Multiple R-squared: 0.554, Adjusted R-squared: 0.521  
F-statistic: 16.8 on 2 and 27 DF, p-value: 1.84e-05

Now compare these results to the ones we get after scaling the area and elevation variables. Remember that the `scale` function centers a variable on zero by subtracting the mean from each observation, and then scales the result by dividing by the standard deviation. This ensures that each regression input has mean 0 and standard deviation 1, and is thus a *z score*.

```
model2.z <- lm(species ~ scale(area) + scale(elevation), data=gala)
summary(model2.z)
```

```
Call:
lm(formula = species ~ scale(area) + scale(elevation), data = gala)

Residuals:
    Min      1Q  Median      3Q     Max 
-192.62 -33.53 -19.20   7.54 261.51 

Coefficients:
            Estimate Std. Error t value Pr(>|t|)    
(Intercept)    85.2      14.5    5.88  2.9e-06 ***
scale(area)     16.2      22.4    0.72  0.4748    
scale(elevation) 72.4      22.4    3.23  0.0032 **  
---
Signif. codes:  0 '***' 0.001 '**' 0.01 '*' 0.05 '.' 0.1 ' ' 1

Residual standard error: 79.3 on 27 degrees of freedom
Multiple R-squared:  0.554, Adjusted R-squared:  0.521 
F-statistic: 16.8 on 2 and 27 DF,  p-value: 1.84e-05
```

### 49.1.1 Questions about the Semi-Standardized Model

79. What changes after centering and rescaling the predictors, and what does not?
80. Why might rescaling like this be a helpful thing to do if you want to compare predictors in terms of importance?

## 49.2 Fully Standardized Regression Coefficients

Suppose we standardize the coefficients by also taking centering and scaling (using the z score) the outcome variable: `species`, creating a **fully standardized** model.

```
model2.zout <- lm(scale(species) ~
                     scale(area) + scale(elevation), data=gala)
summary(model2.zout)
```

```
Call:
lm(formula = scale(species) ~ scale(area) + scale(elevation),
    data = gala)

Residuals:
    Min      1Q  Median      3Q     Max 
-1.6803 -0.2925 -0.1675  0.0658  2.2813 

Coefficients:
```

```

            Estimate Std. Error t value Pr(>|t|)
(Intercept) 4.59e-17 1.26e-01    0.00  1.0000
scale(area)  1.42e-01 1.96e-01    0.72  0.4748
scale(elevation) 6.32e-01 1.96e-01    3.23  0.0032 **
---
Signif. codes: 0 '***' 0.001 '**' 0.01 '*' 0.05 '.' 0.1 ' ' 1

Residual standard error: 0.692 on 27 degrees of freedom
Multiple R-squared: 0.554, Adjusted R-squared: 0.521
F-statistic: 16.8 on 2 and 27 DF, p-value: 1.84e-05

```

### 49.2.1 Questions about the Standardized Model

81. How do you interpret the value 0.142 of the `scale(area)` coefficient here? You may want to start by reviewing the summary of the original `gala` data shown here.

```
summary(gala[c("species", "area", "elevation")])
```

species	area	elevation
Min. : 2	Min. : 0	Min. : 25
1st Qu.: 13	1st Qu.: 0	1st Qu.: 98
Median : 42	Median : 3	Median : 192
Mean : 85	Mean : 262	Mean : 368
3rd Qu.: 96	3rd Qu.: 59	3rd Qu.: 435
Max. :444	Max. :4669	Max. :1707

82. How do you interpret the value 0.632 of the `scale(elevation)` coefficient in the standardized model?  
 83. What is the intercept in this setting? Will this be the case whenever you scale like this?  
 84. What are some of the advantages of looking at scaled regression coefficients?  
 85. Why are these called *fully* standardized coefficients while the previous page described semi-standardized coefficients?  
 86. What would motivate you to use one of these two methods of standardization (fully standardized or semi-standardized) vs. the other?

## 49.3 Robust Standardization of Regression Coefficients

Another common option for scaling is to specify lower and upper comparison points, perhaps by comparing the impact of a move from the 25th to the 75th percentile for each variable, while holding all of the other variables constant.

Occasionally, you will see robust semi-standardized regression coefficients, which measure the increase in the outcome, Y, associated with an increase in that particular predictor of one IQR (inter-quartile range).

```

gala$area.scaleiqr <- (gala$area - mean(gala$area)) / IQR(gala$area)
gala$elevation.scaleiqr <- (gala$elevation - mean(gala$elevation)) /
                           IQR(gala$elevation)

model2.iqr <- lm(species ~ area.scaleiqr + elevation.scaleiqr,
                  data=gala)
summary(model2.iqr)

```

```

Call:
lm(formula = species ~ area.scaleiqr + elevation.scaleiqr, data = gala)

```

Residuals:

Min	1Q	Median	3Q	Max
-192.62	-33.53	-19.20	7.54	261.51

Coefficients:

	Estimate	Std. Error	t value	Pr(> t )
(Intercept)	85.23	14.48	5.88	2.9e-06 ***
area.scaleiqr	1.11	1.53	0.72	0.4748
elevation.scaleiqr	57.96	17.95	3.23	0.0032 **

---

Signif. codes: 0 '\*\*\*' 0.001 '\*\*' 0.01 '\*' 0.05 '.' 0.1 ' ' 1

Residual standard error: 79.3 on 27 degrees of freedom

Multiple R-squared: 0.554, Adjusted R-squared: 0.521

F-statistic: 16.8 on 2 and 27 DF, p-value: 1.84e-05

### 49.3.1 Questions about Robust Standardization

87. How should we interpret the 57.96 value for the scaled `elevation` variable? You may want to start by considering the summary of the original elevation data below.

```
summary(gala$elevation)
```

Min.	1st Qu.	Median	Mean	3rd Qu.	Max.
25	98	192	368	435	1707

A **robust standardized coefficient** analysis measures the increase in Y (in IQR of Y) associated with an increase in the predictor of interest of one IQR.

```
gala$species.scaleiqr <- (gala$species - mean(gala$species)) / IQR(gala$species)
model2.iqrout <- lm(species.scaleiqr ~ area.scaleiqr + elevation.scaleiqr, data=gala)
model2.iqrout
```

Call:

```
lm(formula = species.scaleiqr ~ area.scaleiqr + elevation.scaleiqr,
  data = gala)
```

Coefficients:

(Intercept)	area.scaleiqr	elevation.scaleiqr
-1.01e-16	1.34e-02	6.98e-01

88. What can we learn from the R output above?

## 49.4 Scaling Inputs by Dividing by 2 Standard Deviations

It turns out that standardizing the inputs to a regression model by dividing by a standard deviation creates some difficulties when you want to include a binary predictor in the model.

Instead, Andrew Gelman recommends that you consider centering all of the predictors (binary or continuous) by subtracting off the mean, and then, for the non-binary predictors, also dividing not by one, but rather by two standard deviations.

- Such a standardization can go a long way to helping us understand a model whose predictors are on different scales, and provides an interpretable starting point.
- Another appealing part of this approach is that in the `arm` library, Gelman and his colleagues have created an R function called `standardize`, which can be used to automate the process of checking coefficients that have been standardized in this manner, after the regression model has been fit.

```
model2
```

```
Call:  
lm(formula = species ~ area + elevation, data = gala)
```

```
Coefficients:  
(Intercept) area elevation  
17.1052 0.0188 0.1717
```

```
arm:::standardize(model2)
```

```
Call:  
lm(formula = species ~ z.area + z.elevation, data = gala)
```

```
Coefficients:  
(Intercept) z.area z.elevation  
85.2 32.5 144.8
```

#### 49.4.1 Questions about Standardizing by Dividing by Two SD

89. How does this result compare to the semi-standardized regression coefficients we have seen on the previous few pages?
90. How should we interpret the `z.area` coefficient of 32.5 here? Again, you may want to start by obtaining a statistical summary of the original `area` data, as shown below.

```
summary(gala$area)
```

Min.	1st Qu.	Median	Mean	3rd Qu.	Max.
0	0	3	262	59	4669

To standardize the outcome in this way, as well, we use

```
arm:::standardize(model2, standardize.y=TRUE)
```

```
Call:  
lm(formula = z.species ~ z.area + z.elevation, data = gala)
```

```
Coefficients:  
(Intercept) z.area z.elevation  
1.65e-19 1.42e-01 6.32e-01
```

91. How should we interpret the `z.area` coefficient of 0.142 here?
92. How does these relate to the standardized regression coefficients we've seen before?

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