

# 023 - Final Review

EPIB 607 - FALL 2020

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slides compiled on November 28, 2020





# Exam Details

- **When:** Friday December 8, 2020. The exam will be made available on Crowdmark as of 9am EST for 48 hours.
- This is a timed assessment. As soon as you download the exam, you will have 6 hours to complete and upload your solutions to Crowdmark. **There will be a 5% per hour lateness penalty.**
- This is an open book exam. Any material on myCourses (EPIB607/613) and personal notes are permitted.
- You are not permitted to use the internet and you must work alone. Using the internet or obtaining help from anyone else is considered Cheating as per [Article 17 of the Code of Student Conduct and Disciplinary Procedures](#)
- Provide units and state your assumptions when applicable. Label axes and write answers in complete sentences when appropriate.
- The format of the exam will follow the assignments and the midterm. That is, you will be required to complete a series of questions in an RMarkdown document and knit to pdf. Your solutions for each question must then be uploaded to Crowdmark. A template will be provided which will also include the questions.
- There will be no live zoom meeting. You can email me should you have questions.

# Topics to be covered

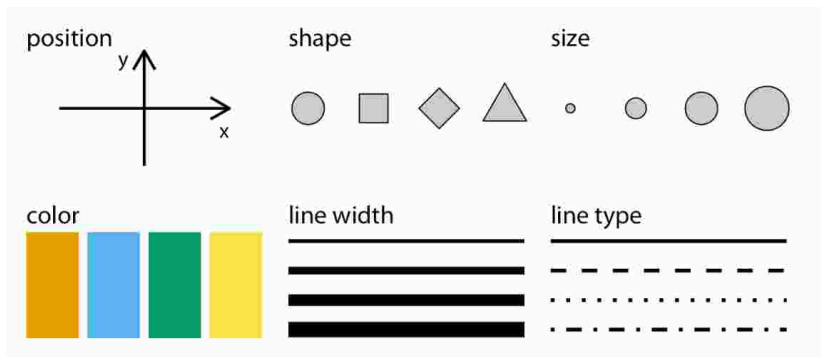
## **Note that the exam is cumulative**

1. Data visualization (histograms, boxplots, scatterplots, line plots), Tidy Data, Color Palettes
2. Descriptive statistics (mean, median, range, IQR, sd, correlation)
3. Normal Curve Calculations, Sampling Distributions, CLT, Bootstrap
4. Confidence intervals, Hypothesis Testing, p-values
5. One sample mean, one sample proportion, one sample rate
6. Power and Sample size calculations
7. Gaussian, Poisson, Binomial regression
8.  $\chi^2$  goodness of fit and contingency tables
9. Permutation testing



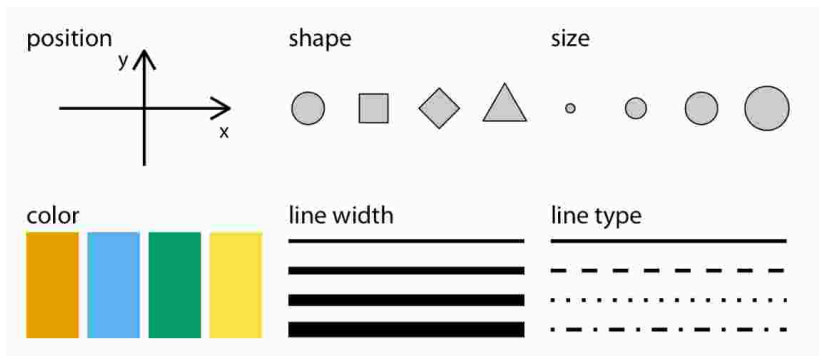
# Aesthetics

- Aesthetics



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- Aesthetics



- Commonly used aesthetics in data visualization: position, shape, size, color, line width, line type. Some of these aesthetics can represent both continuous and discrete data (position, size, line width, color) while others can only represent discrete data (shape, line type)

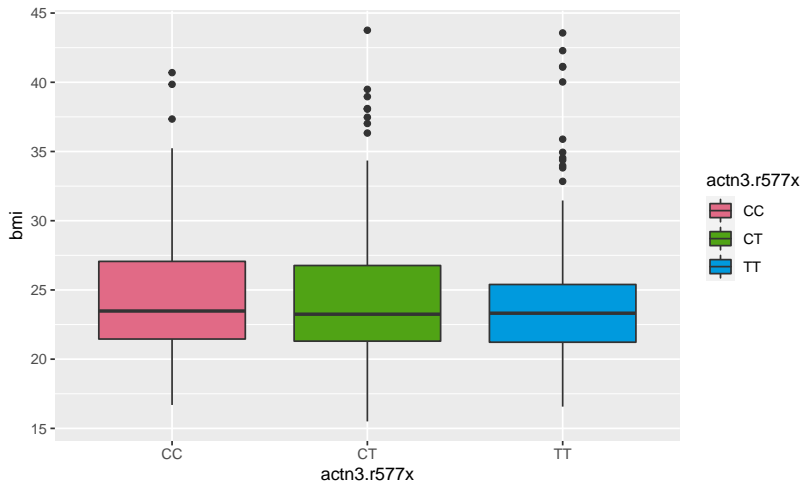
# Types of Graphs

- Review the types of graphs created in the assignments.
- You should be able to critique a graph and propose appropriate graphics for a given dataset. Be mindful of the research question. The graphic should try to answer the research question.
- <https://serialmentor.com/dataviz/directory-of-visualizations.html>
- <https://www.data-to-viz.com/>



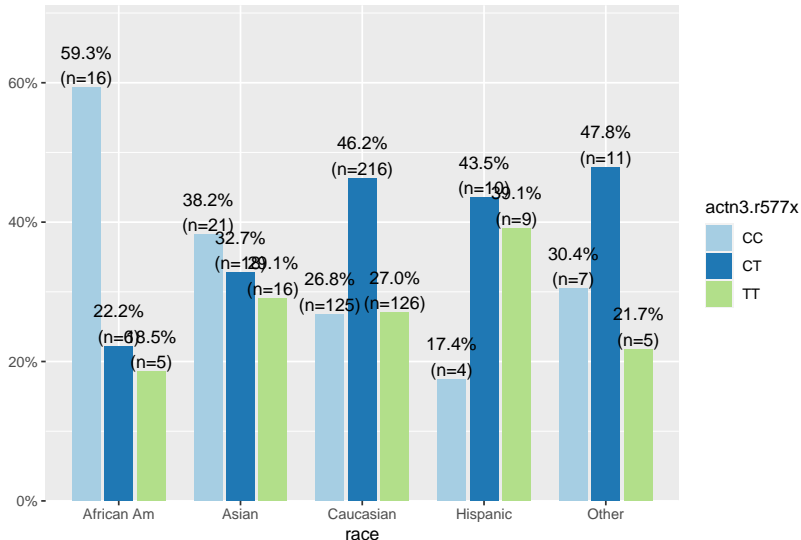
# Boxplots with qualitative palette

```
library(oibistat); data("famuss")  
library(ggplot2)  
library(colorspace)  
  
ggplot(famuss, aes(x = actn3.r577x, y = bmi, fill = actn3.r577x)) +  
  geom_boxplot() +  
  colorspace::scale_fill_discrete_qualitative()
```



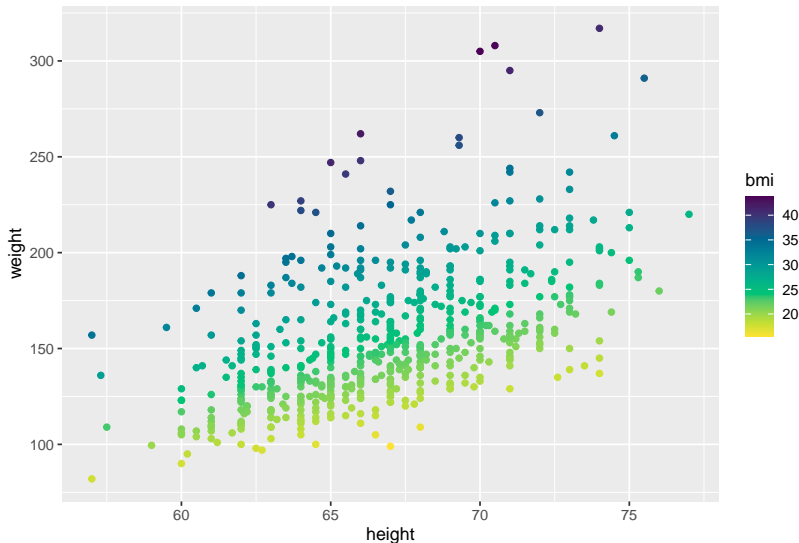
# Conditional distribution of genotype *given* race

```
sjPlot::plot_xtab(famuss$race, famuss$actn3.r577x, margin = "row")
```



# Scatter plots with sequential palette

```
ggplot(famuss, aes(x = height, y = weight, color = bmi)) +  
  geom_point() +  
  colorspace::scale_color_continuous_sequential(palette = "Viridis")
```

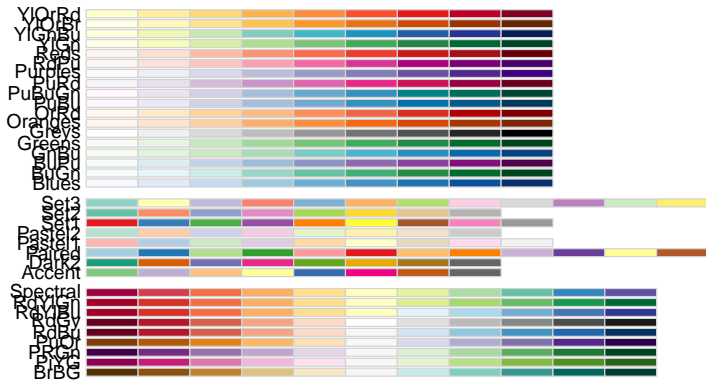


# Variable Types

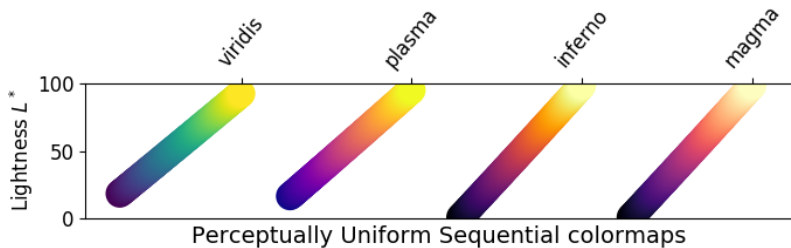
- quantitative/numerical continuous (1.3, 5.7, 83,  $1.5 \times 10^{-2}$ )
- quantitative/numerical discrete (1,2,3,4)
- qualitative/categorical unordered (dog, cat, fish)
- qualitative/categorical ordered (good, fair, poor)

# Color Palettes: Cynthia Brewer

```
pacman::p_load(RColorBrewer)
RColorBrewer::display.brewer.all()
```



# Color Palettes: viridis



# Tidy data

- Each variable forms a column.
- Each observation forms a row.
- Each type of observational units forms a table
- Tidy data is ready for regression routines and plotting

country	year	cases	population
Afghanistan	1999	178	19997071
Afghanistan	2000	1666	200095360
Brazil	1999	30737	172006362
Brazil	2000	80488	174004898
China	1999	211258	1272015272
China	2000	217066	1280008583

variables

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values

# Example: Is it tidy?

MODE OF DELIVERY	COVARIATE			No. OF MOTHER- CHILD PAIRS	No. OF HIV-1- INFECTED CHILDREN
	NO. OF PERIODS OF ANTIRETROVIRAL THERAPY	ADVANCED MATERNAL DISEASE	LOW BIRTH WEIGHT OF INFANT (<2500 g)		
Elective cesarean	0	No	No	372	30
Other	0	No	No	3850	652
Elective cesarean	0	Yes	No	28	5
Other	0	Yes	No	303	74
Elective cesarean	0	No	Yes	110	17
Other	0	No	Yes	767	196
Elective cesarean	0	Yes	Yes	27	4
Other	0	Yes	Yes	114	40
Elective cesarean	1 or 2	No	No	41	0
Other	1 or 2	No	No	441	49
Elective cesarean	1 or 2	Yes	No	23	3
Other	1 or 2	Yes	No	186	33
Elective cesarean	1 or 2	No	Yes	7	0
Other	1 or 2	No	Yes	83	22
Elective cesarean	1 or 2	Yes	Yes	10	3
Other	1 or 2	Yes	Yes	54	19
Elective cesarean	3	No	No	124	2
Other	3	No	No	878	49
Elective cesarean	3	Yes	No	34	1
Other	3	Yes	No	208	24
Elective cesarean	3	No	Yes	25	0
Other	3	No	Yes	109	11
Elective cesarean	3	Yes	Yes	8	1
Other	3	Yes	Yes	28	6



# Descriptive statistics

- Boxplots, histograms, density plot
- IQR, median, mode, mean, min, max, range
- Q1, Q3
- Skewness (long left/right tail)
- Correlation

# Descriptive stats by group

```
library(oibistat); data("famuss")
library(dplyr)

famuss %>%
  dplyr::group_by(actn3.r577x) %>%
  dplyr::summarise(mean_bmi = mean(bmi),
                    sd_bmi = sd(bmi))

## # A tibble: 3 x 3
##   actn3.r577x mean_bmi sd_bmi
##   <fct>         <dbl> <dbl>
## 1 CC           24.5    4.41
## 2 CT           24.5    4.55
## 3 TT           24.2    4.81
```

# Subsetting data

```
library(oibiostat); data("famuss")
library(dplyr)

f.male <- famuss %>%
  dplyr::filter(sex == "Male")

f.male.cauc <- famuss %>%
  dplyr::filter(sex == "Male" & race == "Caucasian")

f.bmi.low <- famuss %>%
  dplyr::filter(bmi <= 23)
```

# Standard error (SE) of a sample statistic

- Recall: When we are talking about the variability of a **statistic**, we use the term **standard error** (not standard deviation). The standard error of the sample mean is  $\sigma/\sqrt{n}$ .

## Remark (SE vs. SD)

*In quantifying the instability of the sample mean ( $\bar{y}$ ) statistic, we talk of SE of the mean (SEM)*

*$SE(\bar{y})$  describes how far  $\bar{y}$  could (typically) deviate from  $\mu$ ;*

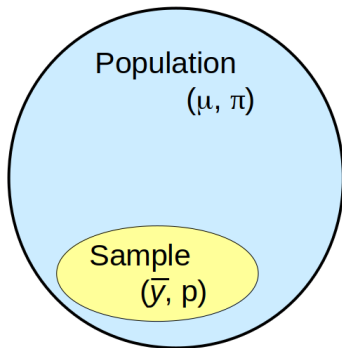
*$SD(y)$  describes how far an individual  $y$  (typically) deviates from  $\mu$  (or from  $\bar{y}$ ).*

# Parameters, Samples, and Statistics

- **Parameter:** An unknown numerical constant pertaining to a population/universe, or in a statistical model.
  - ▶  $\mu$ : population mean                       $\pi$ : population proportion
- **Statistic:** A numerical quantity calculated from a sample. The empirical counterpart of the parameter, used to *estimate* it.
  - ▶  $\bar{y}$ : sample mean                       $p$ : sample proportion

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# Samples must be random

- The validity of inference will depend on the way that the sample was collected. If a sample was collected badly, no amount of statistical sophistication can rescue the study.
- Samples should be **random**. That is, there should be no systematic set of characteristics that is related to the scientific question of interest that causes some people to be more likely to be sampled than others. The simplest type of randomization selects members from the population with equal probability (a uniform distribution).

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- **Do not cheat by**
  - ▶ Taking 5 people from the same household to estimate
    - ▶ proportion of Québécois who don't have a family doctor
    - ▶ who saw a medical doctor last year
    - ▶ average rent
  - ▶ Sampling the depth of the ocean only around Montreal to estimate
    - ▶ proportion of Earth's surface covered by water

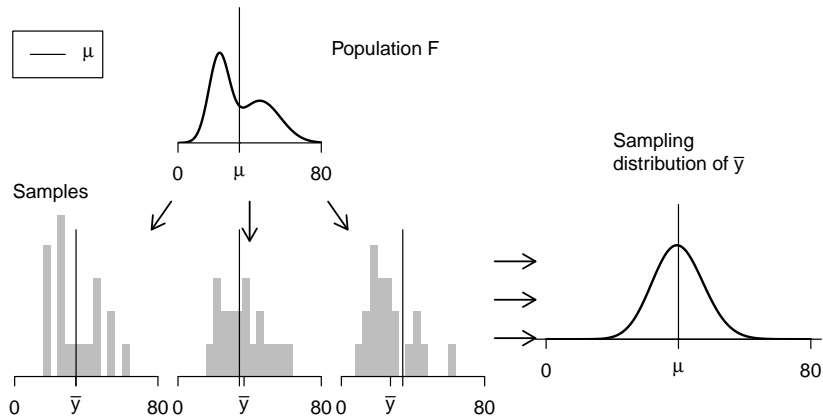


# Sampling Distributions

## Definition (Sampling Distribution)

- *The sampling distribution of a statistic is the distribution of values taken by the statistic in **all possible samples of the same size** from the same population.*
- *The standard deviation of a sampling distribution is called a **standard error***

# Sampling Distributions



**Figure:** Ideal world. Sampling distributions are obtained by drawing repeated samples from the population, computing the statistic of interest for each, and collecting (an infinite number of) those statistics as the sampling distribution

# Why are sampling distributions important?

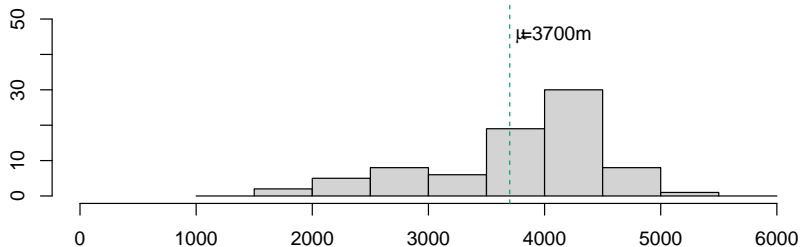
- They tell us how far from the target (true value of the parameter) our statistical shot at it (i.e. the statistic calculated from a sample) is likely to be, or, to have been.

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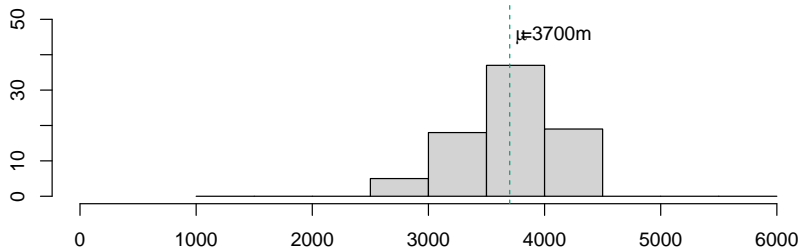
- They tell us how far from the target (true value of the parameter) our statistical shot at it (i.e. the statistic calculated from a sample) is likely to be, or, to have been.
- Thus, they are used in confidence intervals for parameters. Specific sampling distributions (based on a null value for the parameter) are also used in statistical tests of hypotheses.

# Sampling distribution: mean depth of the ocean

**n = 5**

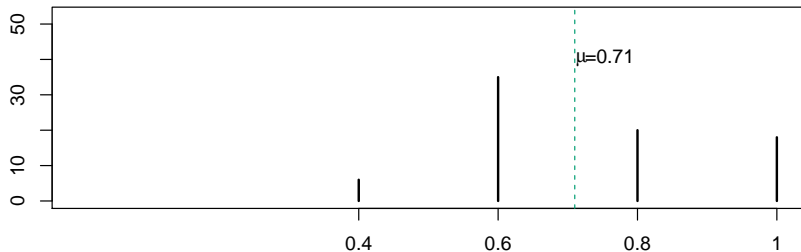


**n = 20**

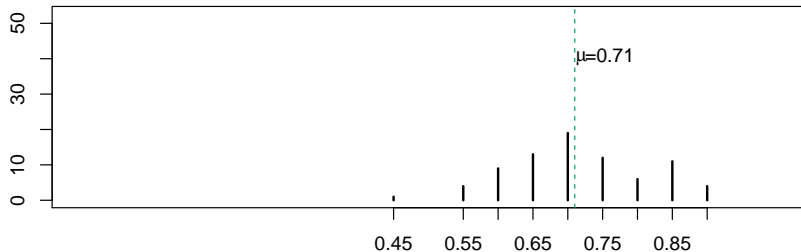


# Sampling distribution: proportion covered by water

**n = 5**



**n = 20**

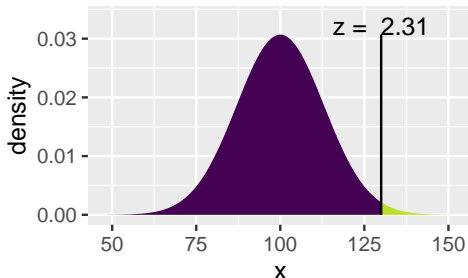


# Normal Distribution: For probabilities we use *pnorm*

```
stats::pnorm(q = 130, mean = 100, sd = 13)
```

```
## [1] 0.99
```

```
mosaic::xpnorm(q = 130, mean = 100, sd = 13)
```



```
## [1] 0.99
```

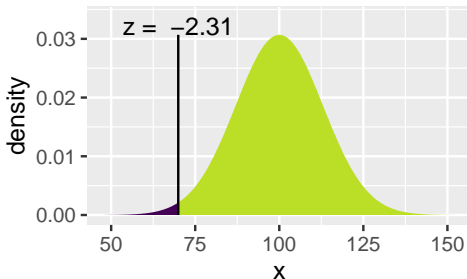
- `pnorm` returns the integral from  $-\infty$  to  $q$  for a  $\mathcal{N}(\mu, \sigma)$
- `pnorm` goes from *quantiles* (think *Z* scores) to probabilities

# Normal Distribution: For quantiles we use *qnorm*

```
stats::qnorm(p = 0.0104, mean = 100, sd = 13)
```

```
## [1] 70
```

```
mosaic::xqnorm(p = 0.0104, mean = 100, sd = 13)
```

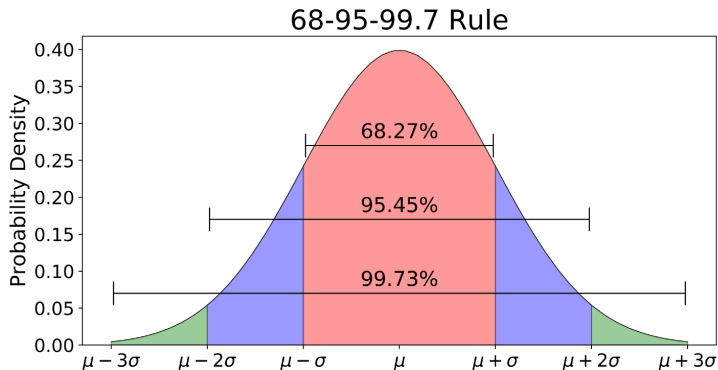


```
## [1] 70
```

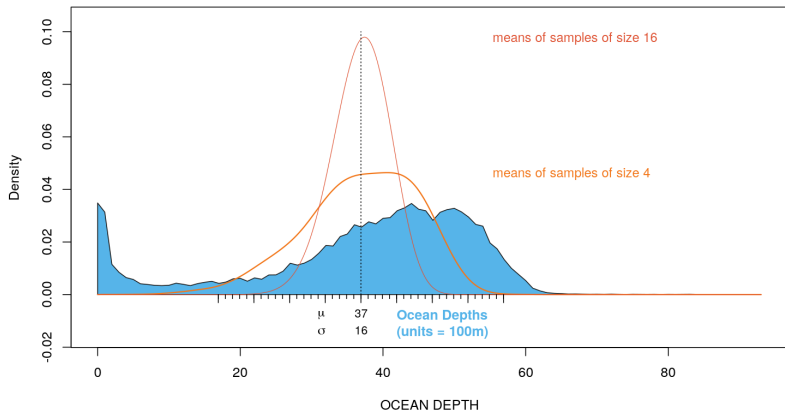
- `qnorm` answers the question: What is the Z-score of the  $p$ th percentile of the normal distribution?
- `qnorm` goes from *probabilities* to quantiles



# Empirical Rule or 68-95-99.7% Rule



# Quadruple the work, half the benefit



**Figure:** When the sample size increases from 4 to 16, the spread of the sampling distribution for the mean is reduced by a half, i.e., the range is cut in half. This is known as the curse of the  $\sqrt{n}$

# The Central Limit Theorem (CLT)

- The sampling distribution of  $\bar{y}$  is, for a large enough  $n$ , close to Gaussian in shape no matter what the shape of the distribution of individual  $Y$  values.
- This phenomenon is referred to as the CENTRAL LIMIT THEOREM
- The CLT applied also to a sample proportion, slope, correlation, or any other statistic created by aggregation of individual observations

## Theorem (Central Limit Theorem)

*if  $Y \sim ???(\mu_Y, \sigma_Y)$ , then*

$$\bar{y} \sim \mathcal{N}(\mu_Y, \sigma_Y/\sqrt{n})$$

# Confidence Interval

## Definition (Confidence Interval)

*A level  $C$  confidence interval for a parameter has two parts:*

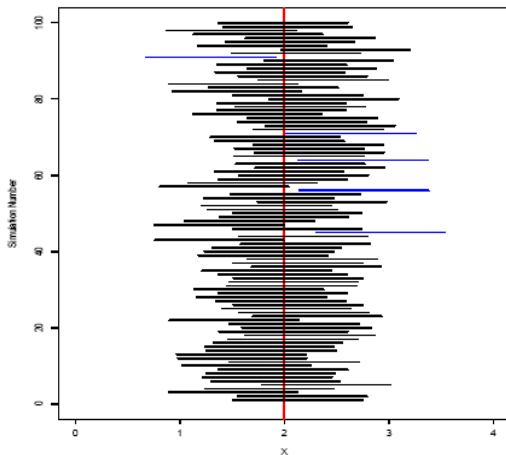
- 1. An interval calculated from the data, usually of the form*

$$\text{estimate} \pm \text{margin of error}$$

*where the estimate is a sample statistic and the margin of error represents the accuracy of our guess for the parameter.*

- 2. A confidence level  $C$ , which gives the probability that the interval will capture the true parameter value in different possible samples. That is, the confidence level is the success rate for the method*

## Confidence Interval: A simulation study



**Figure:** True parameter value is 2 (red line). Each horizontal black line represents a 95% CI from a sample and contains the true parameter value. The blue CIs do not contain the true parameter value. 95% of all samples give an interval that contains the population parameter.

# Interpreting a frequentist confidence interval

- The confidence level is the success rate of the method that produces the interval.

# Interpreting a frequentist confidence interval

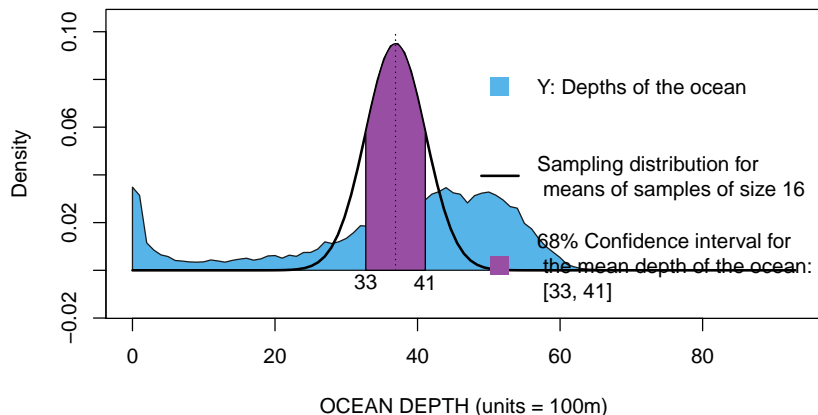
- The confidence level is the success rate of the method that produces the interval.
- We don't know whether the 95% confidence interval from a particular sample is one of the 95% that capture  $\theta$  (the unknown population parameter), or one of the unlucky 5% that miss.

# Interpreting a frequentist confidence interval

- The confidence level is the success rate of the method that produces the interval.
- We don't know whether the 95% confidence interval from a particular sample is one of the 95% that capture  $\theta$  (the unknown population parameter), or one of the unlucky 5% that miss.
- To say that we are 95% confident that the unknown value of  $\theta$  lies between  $U$  and  $L$  is shorthand for “We got these numbers using a method that gives correct results 95% of the time.”



## 68% Confidence interval using qnorm



**Figure:** 68% Confidence interval calculated using `qnorm(p = c(0.16,0.84), mean = 37, sd = 4.2)`

## 95% Confidence interval using qnorm

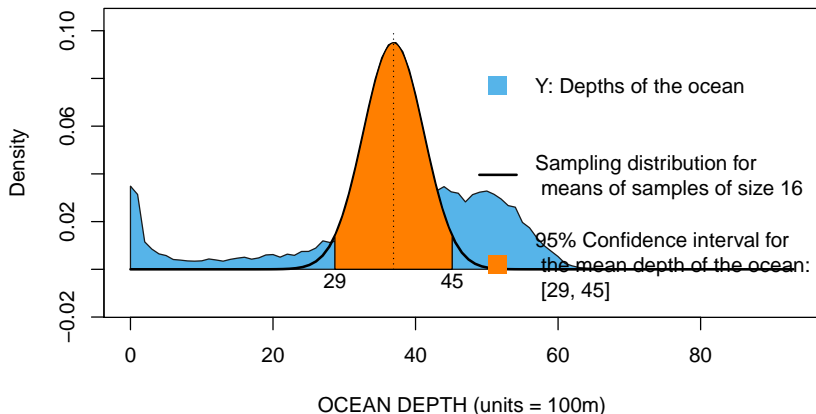


Figure: 95% Confidence interval calculated using `qnorm(p = c(0.025, 0.975), mean = 37, sd = 4.2)`

## Example: Inference for a single population mean

So what does the CI allow us to learn about  $\mu$ ??

- It tells us that if we repeated this procedure again and again (collecting a sample mean, and constructing a 95% CI), 95% of the time, the CI would *cover*  $\mu$ .

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- That is, with 95% probability, the *procedure* will include the true value of  $\mu$ . Note that we are making a probability statement about the CI, not about the parameter.
- Unfortunately, we do not know whether the true value of  $\mu$  is contained in the CI in the particular experiment that we have performed.

# Motivation for the Bootstrap

- The  $\pm$  and qnorm methods to calculate a CI both require the CLT

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Q: What happens if there is no formula available to calculate a CI?



# Motivation for the Bootstrap

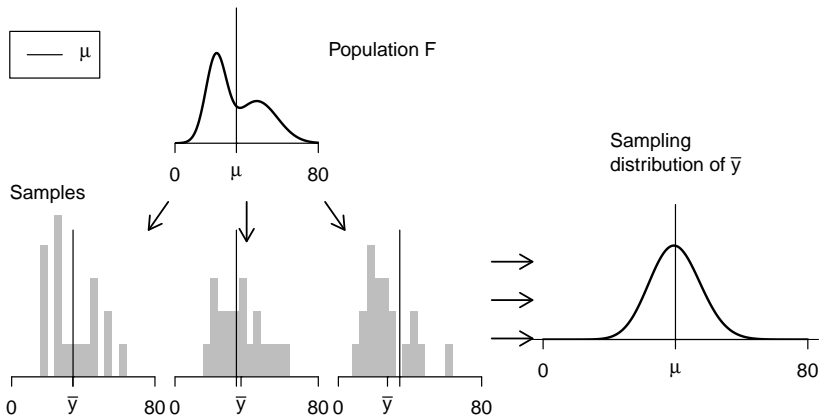
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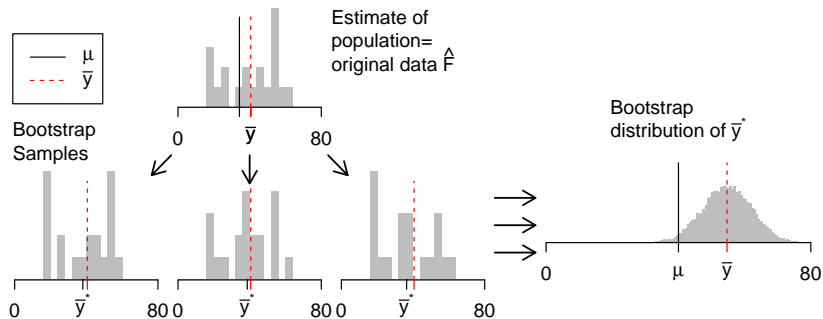
A: Bootstrap

# Ideal world: known sampling distribution



**Figure:** Ideal world. Sampling distributions are obtained by drawing repeated samples from the population, computing the statistic of interest for each, and collecting (an infinite number of) those statistics as the sampling distribution

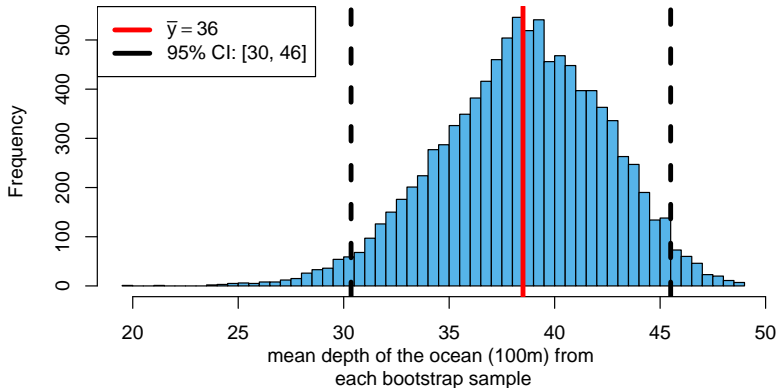
# Reality: use the bootstrap distribution instead



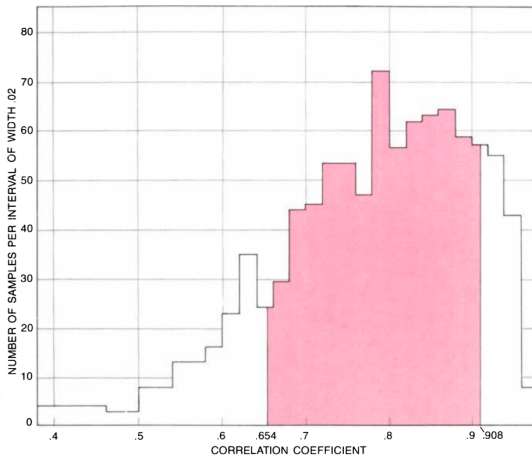
**Figure:** Bootstrap world. The bootstrap distribution is obtained by drawing repeated samples from an estimate of the population, computing the statistic of interest for each, and collecting those statistics. The distribution is centered at the observed statistic ( $\bar{y}$ ), not the parameter ( $\mu$ ).

# Main idea: simulate your own sampling distribution

```
R <- replicate(B, {  
  dplyr::sample_n(depths.n.20, size = N, replace = TRUE) %>%  
  dplyr::summarize(r = mean(alt)) %>%  
  dplyr::pull(r)  
})  
CI_95 <- quantile(R, probs = c(0.025, 0.975))
```



# Bootstrap can be used for other statistics (e.g. $R^2$ )



source: Bootstrap article in Scientific American

## $\sigma$ known vs. unknown

$\sigma$	known	unknown
Data	$\{y_1, y_2, \dots, y_n\}$	$\{y_1, y_2, \dots, y_n\}$
Pop'n param	$\mu$	$\mu$
Estimator	$\bar{y} = \frac{1}{n} \sum_{i=1}^n y_i$	$\bar{y} = \frac{1}{n} \sum_{i=1}^n y_i$
SD	$\sigma$	$s = \sqrt{\frac{\sum_{i=1}^n (y_i - \bar{y})^2}{n-1}}$
SEM	$\sigma / \sqrt{n}$	$s / \sqrt{n}$
$(1 - \alpha)100\%$ CI	$\bar{y} \pm z_{1-\alpha/2}^*(\text{SEM})$	$\bar{y} \pm t_{1-\alpha/2, (n-1)}^*(\text{SEM})$
test statistic	$\frac{\bar{y} - \mu_0}{\text{SEM}} \sim \mathcal{N}(0, 1)$	$\frac{\bar{y} - \mu_0}{\text{SEM}} \sim t_{(n-1)}$

# Assumptions

	$z$	$t$	Bootstrap
SRS	✓	✓	✓
Normal population	✓*	✓*	✗
needs CLT	✓*	✓*	✗
$\sigma$ known	✓	✗	✗
Sampling dist. center at	$\mu$	$\mu$	$\bar{y}$
SD	$\sigma$	$s$	$s$
SEM	$\sigma/\sqrt{n}$	$s/\sqrt{n}$	SD(bootstrap statistics)

---

<sup>a\*</sup>If population is Normal then CLT is not needed. If population is not Normal then CLT is needed.

- Binomial calculations
- Nomogram, Clopper-Pearson CI
- Normal approximation



# $p$ -values and statistical tests

## Definition ( $p$ -value)

A **probability concerning the observed data**, calculated under a **Null Hypothesis** assumption, i.e., assuming that the only factor operating is sampling or measurement variation.

Use To assess the evidence provided by the sample data in relation to a pre-specified claim or ‘hypothesis’ concerning some parameter(s) or data-generating process.

Basis As with a confidence interval, it makes use of the concept of a *distribution*.

Caution A  $p$ -value is NOT the probability that the null ‘hypothesis’ is true

## More about the $p$ -value

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- The  $p$ -value is a **probability concerning data, conditional on the Null Hypothesis being true**.
- **It is not the probability that Null Hypothesis is true**, *conditional on the data*.

$$\begin{aligned} p_{\text{value}} &= P(\text{this or more extreme data} | H_0) \\ &\neq P(H_0 | \text{this or more extreme data}). \end{aligned}$$

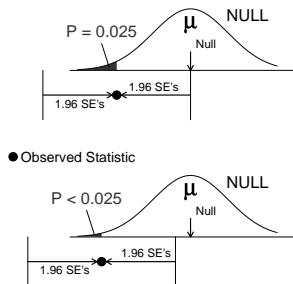
# More about the $p$ -value

- The  $p$ -value is a **probability concerning data, conditional on the Null Hypothesis being true.**
- **It is not the probability that Null Hypothesis is true, conditional on the data.**

$$p_{\text{value}} = P(\text{this or more extreme data} | H_0) \\ \neq P(H_0 | \text{this or more extreme data}).$$

- Statistical tests are often coded as statistically significant or not according to whether results are extreme or not with respect to a reference (null) distribution. But a test result is just one piece of data, and needs to be considered *along with rest of evidence* before coming to a ‘conclusion.’
- **Likewise with statistical ‘tests’: the  $p$ -value is just one more piece of evidence, hardly enough to ‘conclude’ anything.**

# Close relationship between $p$ -value and CI



- (Upper graph) If upper limit of 95% CI *just touches* null value, then the 2 sided  $p$ -value is 0.05 (or 1 sided  $p$ -value is 0.025).
- (Lower graph) If upper limit *excludes* null value, then the 2 sided  $p$ -value is less than 0.05 (or 1 sided  $p$ -value is less than 0.025).
- (Graph not shown) If CI *includes* null value, then the 2-sided  $p$ -value is greater than (the conventional) 0.05, and thus observed statistic is “not statistically significantly different” from hypothesized null value.

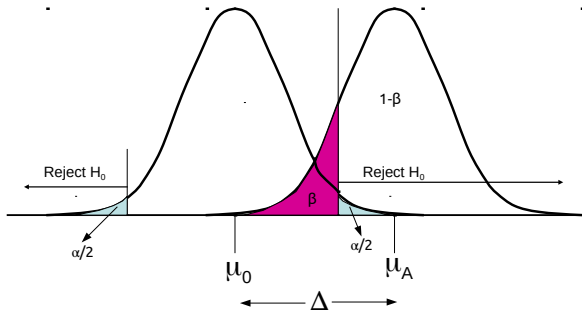
$$\text{Power} = 1 - \beta$$

### Definition (Power = $1 - \beta$ )

The probability that a fixed level  $\alpha$  significance test will reject  $H_0$  when a particular alternative value of the parameter is true is called the **power** of the test to detect the alternative.

Distribution of  $\bar{y}$  under  
the null hypothesis:

Distribution of  $\bar{y}$  under  
an alternative hypothesis:



## Power and Sample Size: 3 questions

1. How much water a supplier could add to the milk before they have a 10% , 50%, 80% chance of getting caught, i.e., of the buyer detecting the cheating ?



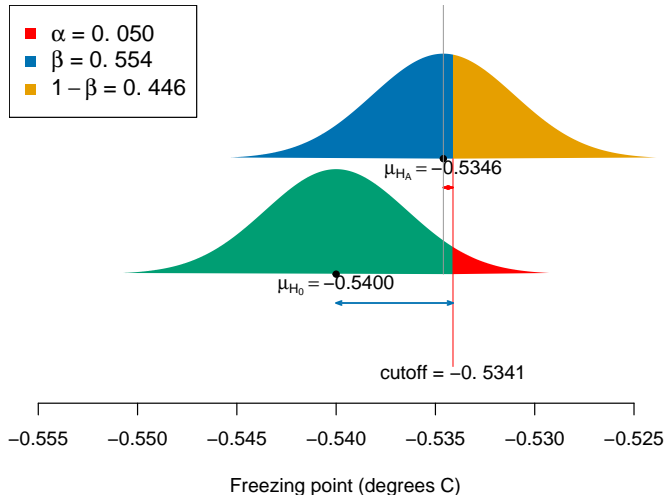
## Power and Sample Size: 3 questions

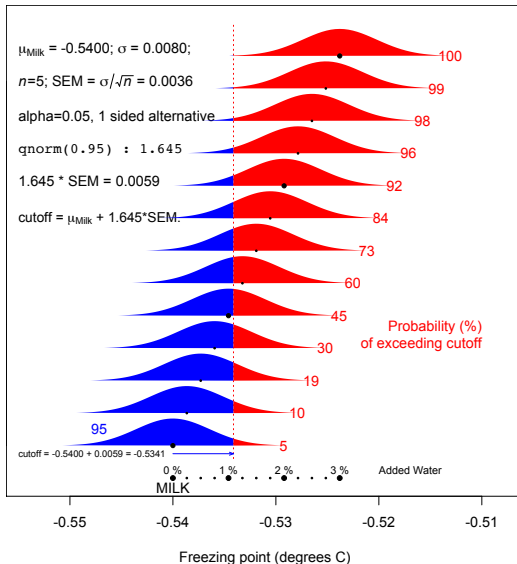
1. How much water a supplier could add to the milk before they have a 10% , 50%, 80% chance of getting caught, i.e., of the buyer detecting the cheating ?
2. Assume a 99:1 mix of milk and water. What are the chances of detecting cheating if the buyer uses samples  $n=10$ , 15 or 20 rather than just 5 measurements?

## Power and Sample Size: 3 questions

1. How much water a supplier could add to the milk before they have a 10% , 50%, 80% chance of getting caught, i.e., of the buyer detecting the cheating ?
2. Assume a 99:1 mix of milk and water. What are the chances of detecting cheating if the buyer uses samples  $n=10, 15$  or  $20$  rather than just 5 measurements?
3. At what  $n$  does the chance of detecting cheating reach 80%? (*a commonly used, but arbitrary, criterion used in sample-size planning by investigators seeking funding for their proposed research*)

If the supplier added 1% water to the milk

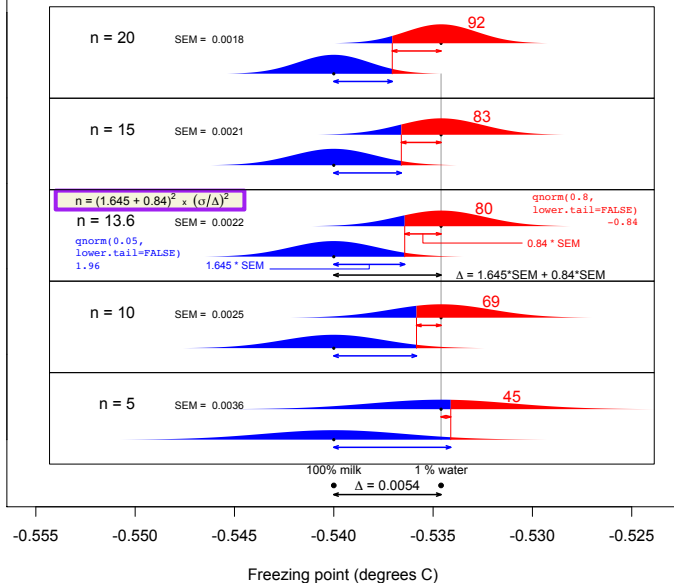




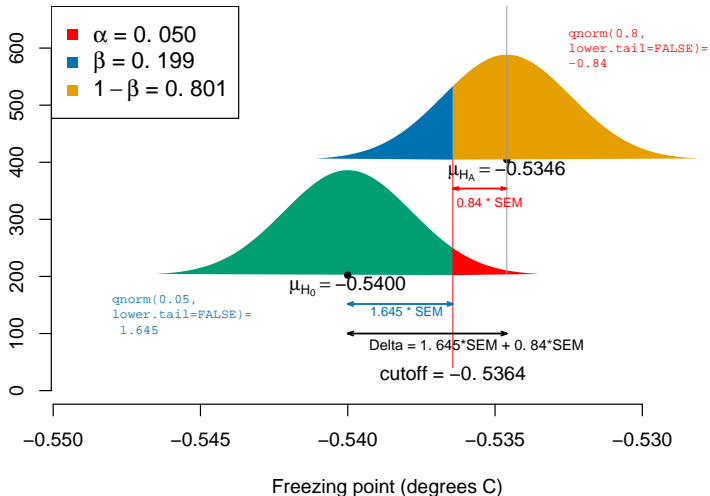
The probabilities in red were calculated using the formula: `stats::pnorm(cutoff, mean = mu.mixture, sd = SEM, lower.tail=FALSE)`

$$\sigma = 0.0080; \text{ SEM} = \sigma/\sqrt{n}$$

$$\text{cutoff} = -0.54 + 1.645 \cdot \text{SEM} \text{ (alpha=0.05, 1 sided alternative)}$$



# The balancing formula



# What sample size needed?

- The 'balancing formula', in SEM terms, is simply the  $n$  where

$$1.645 \times SEM + 0.84 \times SEM = \Delta.$$

Replacing each of the SEMs (assumed equal, because we assumed the variability is approx. the same under both scenarios) by  $\sigma/\sqrt{n}$ , i.e.,

$$1.645 \times \sigma/\sqrt{n} + 0.84 \times \sigma/\sqrt{n} = \Delta.$$

and solving for  $n$ , one gets

$$n = (1.645 + 0.84)^2 \times \left\{ \frac{\sigma}{\Delta} \right\}^2 = (1.645 + 0.84)^2 \times \left\{ \frac{\text{Noise}}{\text{Signal}} \right\}^2.$$

# What sample size needed? General Formula

- Two sided alternative:

$$\Delta = z_{1-\alpha/2} \times SEM + z_{1-\beta} \times SEM$$

- One sided alternative:

$$\Delta = z_{1-\alpha} \times SEM + z_{1-\beta} \times SEM$$





# The Poisson Distribution

- The (infinite number of) probabilities  $P_0, P_1, \dots, P_y, \dots$ , of observing  $Y = 0, 1, 2, \dots, y, \dots$  events in a given amount of “experience.”
- These probabilities,  $P(Y = k) \rightarrow \text{dpois}()$ , are governed by a single parameter, the mean  $E[Y] = \mu$  which represents the expected **number** of events in the amount of experience actually studied.
- We say that a random variable  $Y \sim \text{Poisson}(\mu)$  distribution if

$$P(Y = k) = \frac{\mu^k}{k!} e^{-\mu}, \quad k = 0, 1, 2, \dots$$

- Note: in `dpois()`  $\mu$  is referred to as `lambda`
- Note the distinction between  $\mu$  and  $\lambda$ 
  - ▶  $\mu$ : expected **number** of events
  - ▶  $\lambda$ : **rate** parameter

## Confidence interval for $\mu$

- If the CLT hasn't kicked in, then the usual CI might not be appropriate:

$$\text{point-estimate} \pm z^* \times \text{standard error}$$

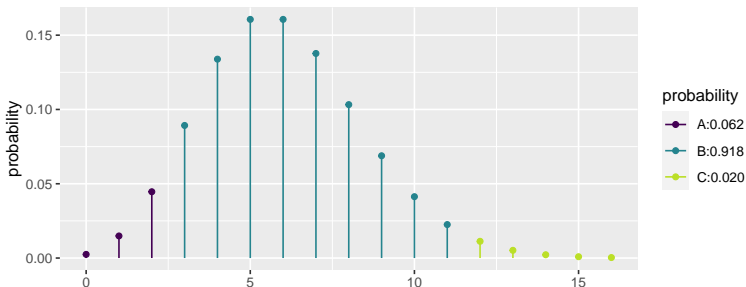
# Confidence interval for $\mu$

- If the CLT hasn't kicked in, then the usual CI might not be appropriate:

$$\text{point-estimate} \pm z^* \times \text{standard error}$$

- `qpois` function doesn't work either:

```
# middle area is not 95%  
mosaic::xqpois(c(0.025, 0.975), lambda = 6)
```



```
## [1] 2 11
```

## Confidence interval for $\mu$

- Similar to the binomial (Clopper-Pearson CI), we consider a *first-principles*  $100(1 - \alpha)\%$  CI  $[\mu_{\text{LOWER}}, \mu_{\text{UPPER}}]$  such that

$$P(Y \geq y \mid \mu_{\text{LOWER}}) = \alpha/2 \quad \text{and} \quad P(Y \leq y \mid \mu_{\text{UPPER}}) = \alpha/2.$$

- For example, the 95% CI for  $\mu$ , based on  $y = 6$ , is  $[\underline{2.20}, \underline{13.06}]$ .
- **Exercise:** can we use `glm` to get a CI for  $\mu$  ?

**LOWER**  
 $\mu = 2.2$

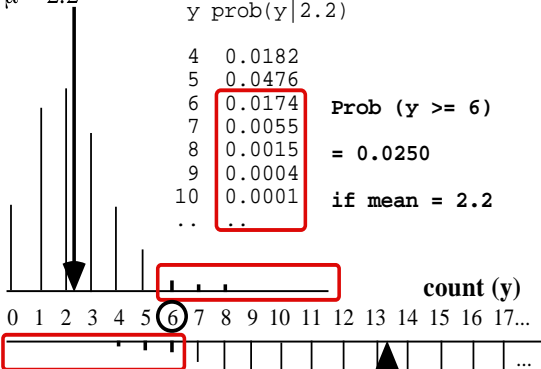
y prob(y|2.2)

4	0.0182
5	0.0476
6	0.0174
7	0.0055
8	0.0015
9	0.0004
10	0.0001
..	..

Prob (y >= 6)

= 0.0250

if mean = 2.2



y prob(y|13.06)

0	0.0000
1	0.0000
2	0.0002
3	0.0008
4	0.0026
5	0.0067
6	0.0147
7	0.0274
..	..

Prob (y <= 6)

= 0.0250

if mean = 13.06

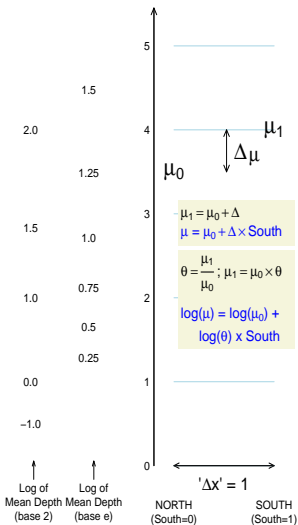
**UPPER**  
 $\mu = 13.06$

⑥ observed count

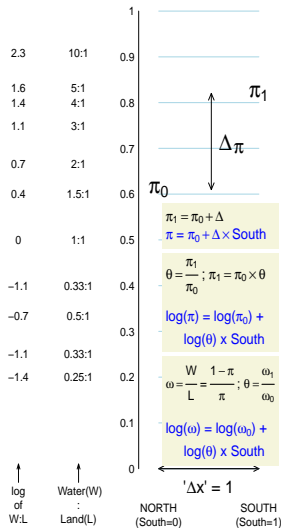
.. ..

$\mu$ 

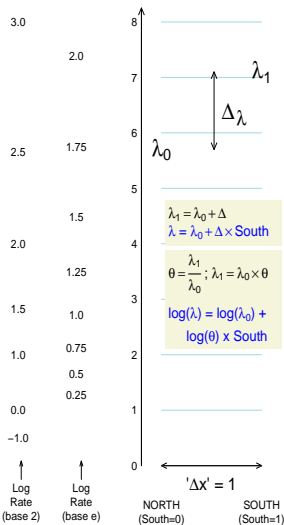
Mean Ocean depth (Km)

fn. of  $\mu_x = \beta_0$  (i.e., this fn. at South = 0) + an additional ' $\beta$ ' if South = 1 $\pi$ 

Proportion Water

fn. of  $\pi_x = \beta_0$  (i.e., this fn. at South = 0) + an additional ' $\beta$ ' if South = 1 $\lambda$ 

Magnitude 6 or higher Earthquakes/Month

fn. of  $\lambda_x = \beta_0$  (i.e., this fn. at South = 0) + an additional ' $\beta$ ' if South = 1

# Deterministic and stochastic model components

- The regression equation specifies the deterministic part of the model.
- This is defined in terms of parameters, conditional on the values of  $X$ .
- To complete the model specification, we need to specify the stochastic component of the model, a statistical distribution for the outcome  $Y_X$ .
- The appropriate distribution is

$$Y_X \sim \text{Gaussian}(\mu_X, \sigma^2)$$

- Here the mean  $\mu_X$  is given by the regression equation as

$$\mu_X = \mu_0 + \Delta_\mu \cdot X$$



```
## Coefficients:
##           Estimate Std. Error t value Pr(>|t|)
## (Intercept)  3628.5      86.5      42  <2e-16
##
## Residual standard error: 1730 on 399 degrees of freedom

fit <- lm(alt ~ South, data = depths); print(summary(fit), signif.stars = F)

## Coefficients:
##           Estimate Std. Error t value Pr(>|t|)
## (Intercept)    3523         122  28.82  <2e-16
## South          211         173   1.22   0.22
##
## Residual standard error: 1730 on 398 degrees of freedom
## Multiple R-squared:  0.00372, Adjusted R-squared:  0.00122
## F-statistic: 1.49 on 1 and 398 DF,  p-value: 0.223

fit <- glm(alt ~ South, data = depths, family = gaussian(link=log)); print(summary(fit), signif.stars = F)

##
## Coefficients:
##           Estimate Std. Error t value Pr(>|t|)
## (Intercept)   8.1671     0.0347  235.41  <2e-16
## South         0.0581     0.0477   1.22   0.22
##
## (Dispersion parameter for gaussian family taken to be 3e+06)
##
## Null deviance: 1193681102  on 399  degrees of freedom
## Residual deviance: 1189239546  on 398  degrees of freedom
## AIC: 7103
##
## Number of Fisher Scoring iterations: 5
```

```
coef(fit)
```

```
## (Intercept)      South  
##      8.167      0.058
```

```
vcov(fit)
```

```
##      (Intercept)      South  
## (Intercept)      0.0012 -0.0012  
## South      -0.0012  0.0023
```

```
confint(fit)
```

```
##      2.5 % 97.5 %  
## (Intercept) 8.097  8.23  
## South      -0.035  0.15
```

## 2.2 Bootstrap CI for mean difference using canned function

```
pacman::p_load(car)
betahat.boot <- car::Boot(fit, R=999)
head(betahat.boot$t)

##      (Intercept) South
## [1,]          8.2 0.060
## [2,]          8.1 0.108
## [3,]          8.1 0.064
## [4,]          8.2 0.060
## [5,]          8.1 0.136
## [6,]          8.1 0.079

dim(betahat.boot$t)

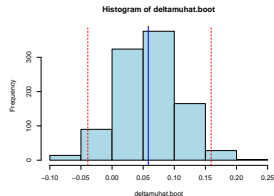
## [1] 999  2

deltamuhat.boot <- betahat.boot$t[,2]
median(deltamuhat.boot)

## [1] 0.058

quantile(deltamuhat.boot, probs = c(0.025, 0.975))

## 2.5%    98%
## -0.039 0.159
```



## 2.2 Bootstrap CI for mean difference using canned function (continued)

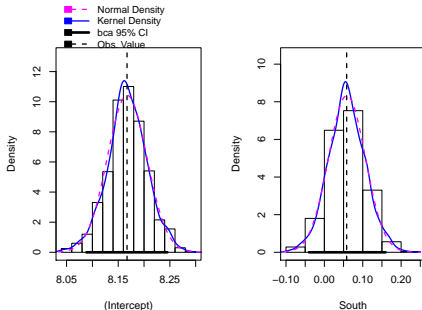
```
summary(betahat.boot)
```

```
##
## Number of bootstrap replications R = 999
##           original bootBias bootSE bootMed
## (Intercept)  8.1671 0.000172 0.0383  8.1666
## South       0.0581 0.000675 0.0478  0.0582
```

```
confint(betahat.boot)
```

```
## Bootstrap bca confidence intervals
##
##           2.5 % 97.5 %
## (Intercept)  8.09  8.25
## South       -0.04  0.16
```

```
hist(betahat.boot)
```



## 2.3 Bootstrap CI for mean difference using boot package

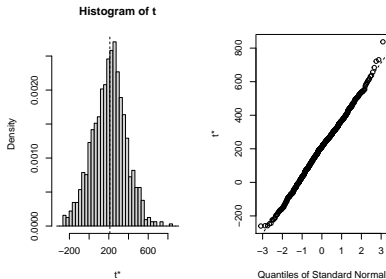
```
plot(results)
```

```
library(boot)
# function to obtain deltamtu hat
deltamu <- function(data, indices) {
  # allows boot to select sample
  d <- data[indices,]
  fit <- lm(alt ~ South, data=d)
  coef(fit)["South"]
}

results <- boot::boot(data = depths,
  statistic = deltamtu, R=999)

results

##
## ORDINARY NONPARAMETRIC BOOTSTRAP
##
##
## Call:
## boot::boot(data = depths, statistic = deltamtu, R
##
##
## Bootstrap Statistics :
##      original bias      std. error
## t1*         211         -6         170
```



```
boot.ci(results)

## BOOTSTRAP CONFIDENCE INTERVAL CALCULATIONS
## Based on 999 bootstrap replicates
##
## CALL :
## boot.ci(boot.out = results)
##
## Intervals :
## Level      Normal              Basic
## 95%   (-116,  549 )   (-112,  570 )
##
## Level      Percentile          BCa
## 95%   (-148,  533 )   (-150,  529 )
## Calculations and Intervals on Original Scale
```

# Permutation Testing

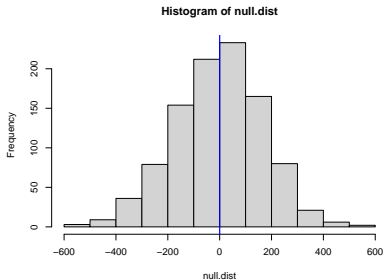
- In testing a null hypothesis we need a test statistic that will have different values under the null hypothesis and the alternatives we care about
- We then need to compute the sampling distribution of the test statistic when the null hypothesis is true. For some test statistics and some null hypotheses this can be done analytically.
- The pvalue is the probability that the test statistic would be at least as extreme as we observed, if the null hypothesis is true.
- A permutation test gives a simple way to compute the sampling distribution for any test statistic, under the null hypothesis that there is no effect (i.e. South is not a determinant of the mean depth of the ocean)
- <https://www.jwilber.me/permutationtest/>

# Permutation Testing

- To estimate the sampling distribution of the test statistic we need many samples generated under the strong null hypothesis.
- If the null hypothesis is true, changing the exposure would have no effect on the outcome. By randomly shuffling the determinants we can make up as many data sets as we like.
- If the null hypothesis is true, the shuffled data sets should look like the real data, otherwise they should look different from the real data.
- The ranking of the real test statistic among the shuffled test statistics gives a p-value

# Permutation Testing

```
one.test <- function(x,y) {  
  xstar <- sample(x)  
  mean(y[xstar==1]) - mean(y[xstar==0])  
}  
  
null.dist <- replicate(1000, one.test(x = depths$South, y = depths$alt))  
hist(null.dist)  
abline(v=coef(fit)["South"], lwd=2, col="blue")
```



```
mean(abs(null.dist) > abs(coef(fit)["South"]))
```

```
## [1] 1
```



# Deterministic and stochastic model components

- The regression equation specifies the deterministic part of the model.
- This is defined in terms of parameters, conditional on the values of  $X$ .
- To complete the model specification, we need to specify the stochastic component of the model, a statistical distribution for the outcome  $Y_X$  (counts).
- The appropriate distribution is

$$Y_X \sim \text{Poisson}(\mu_X)$$

- Here the mean  $\mu_X$  is given by the regression equation as

$$\mu_X = \lambda \cdot \text{PT}$$

See the 2018 Lancet article *Efficacy of Olyset Duo, a bednet containing pyriproxyfen and permethrin, versus a permethrin-only net against clinical malaria in an area with highly pyrethroid-resistant vectors in rural Burkina Faso: a cluster-randomised controlled trial* by Tiono et. al. Reproduce the Rate ratio (95% CI) in Table 2. Calculate the rate difference and 95% CI comparing PPF-treated to Standard long-lasting insecticidal nets. Check the goodness of fit.

```
## Call:
## glm(formula = cases ~ exposure + offset(log(years)), family = poisson(link = log),
##      data = df)
##
## Coefficients:
##              Estimate Std. Error z value Pr(>|z|)
## (Intercept)   0.6831     0.0243   28.09 < 2e-16
## exposure     -0.2669     0.0329   -8.12 4.6e-16
##
## (Dispersion parameter for poisson family taken to be 1)
##
##      Null deviance: 1381.2  on 23  degrees of freedom
## Residual deviance: 1316.0  on 22  degrees of freedom
## AIC: 1477
##
## Number of Fisher Scoring iterations: 5
```

# Deterministic and stochastic model components

- The regression equation specifies the deterministic part of the model.
- This is defined in terms of parameters, conditional on the values of  $Z$  and  $X$ .
- To complete the model specification, we need to specify the stochastic component of the model, a statistical distribution for the outcome  $D_{ZX}$ .
- It is already obvious that the appropriate distribution is

$$D_{ZX} \sim \text{Binomial}(N_{ZX}, \pi_{ZX})$$

- Here the risk  $\pi_{ZX}$  is given by the regression equation as (verify)

$$\pi_{ZX} = \frac{e^{\alpha + \beta Z + \gamma X}}{1 + e^{\alpha + \beta Z + \gamma X}} = \frac{1}{1 + e^{-(\alpha + \beta Z + \gamma X)}}$$

- This inverse transformation is the so-called *expit* function:

$$\pi_{ZX} = \text{logit}^{-1}(\alpha + \beta Z + \gamma X) = \text{expit}(\alpha + \beta Z + \gamma X)$$

# Regression equation with logit link

- Reparametrizing the log-odds is referred to as logistic regression.
- In the ongoing example we may take

$$\log \left( \frac{\pi_{ZX}}{1 - \pi_{ZX}} \right) = \alpha + \beta Z + \gamma X$$

- The original four parameters are now expressed in terms of three new parameters: an intercept term  $\alpha$  and regression coefficients  $\beta$  and  $\gamma$ .
- The function  $\log \frac{\pi}{1-\pi}$  is referred to as the logit transformation of the risk parameter  $\pi$ .
- Thus, the same model can be specified as a reparametrization of the risk parameter together with the *logit link* function:

$$\text{logit}(\pi_{ZX}) = \alpha + \beta Z + \gamma X$$

# Log-linear model for risk

- Is there some particular reason why we *have* to use the logit link when modeling risk?
- Why could we not just parametrize the log-risk as

$$\log(\pi_{ZX}) = \alpha + \beta Z + \gamma X?$$

- We can; in this case the regression coefficient  $\beta$  would be interpreted as a log-risk ratio:

$$\begin{aligned}\frac{\pi_{1X}}{\pi_{0X}} &= \frac{e^{\alpha+\beta+\gamma X}}{e^{\alpha+\gamma X}} \\ &= \frac{e^{\alpha} e^{\beta} e^{\gamma X}}{e^{\alpha} e^{\gamma X}} \\ &= e^{\beta}\end{aligned}$$

$$\Leftrightarrow \log\left(\frac{\pi_{1X}}{\pi_{0X}}\right) = \beta$$

# Contingency Tables and Difference in Proportions

- The function `prop.test()` is used to conduct a hypothesis test for a single proportion or for the difference of two proportions, under the assumption that the sampling distribution for each sample proportion is approximately normal.
- The function `binom.test()` is used to conduct a hypothesis test for a single proportion based on exact binomial probabilities.
- `chisq.test()` performs chi-squared contingency table tests and goodness-of-fit tests. See Vu and Harrington section 8.3

# Session Info

```
R version 4.0.2 (2020-06-22)
Platform: x86_64-pc-linux-gnu (64-bit)
Running under: Pop!_OS 20.04 LTS

Matrix products: default
BLAS:   /usr/lib/x86_64-linux-gnu/openblas-pthread/libblas.so.3
LAPACK: /usr/lib/x86_64-linux-gnu/openblas-pthread/liblapack.so.3

attached base packages:
[1] tools      stats      graphics  grDevices  utils      datasets  methods
[8] base

other attached packages:
[1] boot_1.3-25      car_3.0-9        carData_3.0-4    latex2exp_0.4.0
[5] RColorBrewer_1.1-2 colorspace_1.4-1  oibioestat_0.2.0  NCStats_0.4.7
[9] FSA_0.8.30       forcats_0.5.0    stringr_1.4.0     dplyr_1.0.2
[13] purrr_0.3.4      readr_1.4.0      tidyr_1.1.2       tibble_3.0.4
[17] ggplot2_3.3.2    tidyverse_1.3.0  knitr_1.30

loaded via a namespace (and not attached):
[1] minqa_1.2.4      TH.data_1.0-10   ellipsis_0.3.1
[4] rio_0.5.16       leaflet_2.0.3    sjlabelled_1.1.7
[7] snakecase_0.11.0 estimability_1.3  ggstance_0.3.4
[10] parameters_0.8.6 ggdendro_0.1.22   fs_1.5.0
[13] rstudioapi_0.13  farver_2.0.3     ggrepel_0.8.2
[16] fansi_0.4.1      mvtnorm_1.1-1    lubridate_1.7.9
[19] xml2_1.3.2       codetools_0.2-16 mosaic_1.7.0
[22] splines_4.0.2    sjmisc_2.8.5     polyclip_1.10-0
[25] jsonlite_1.7.1   nloptr_1.2.2.2    ggeffects_0.16.0
[28] broom_0.7.2      dbplyr_1.4.4     ggforce_0.3.2
[31] effectsize_0.3.3 compiler_4.0.2    httr_1.4.2
[34] sjstats_0.18.0   emmeans_1.5.1     backports_1.2.0
[37] assertthat_0.2.1 Matrix_1.2-18     cli_2.1.0
[40] tweenr_1.0.1     htmltools_0.5.0   coda_0.19-4
[43] gtable_0.3.0     glue_1.4.2        Rcpp_1.0.5
[46] cellranger_1.1.0 vctrs_0.3.4       sjPlot_2.8.5
[49] nlme_3.1-149     crosstalk_1.1.0.1 insight_0.9.6
[52] xfun_0.19        ps_1.4.0          lme4_1.1-23
[55] openxlsx_4.1.5   rvest_0.3.6       lifecycle_0.2.0
[58] mosaicCore_0.8.0 pacman_0.5.1      statmod_1.4.34
[61] MASS_7.3-53      zoo_1.8-8         scales_1.1.1
```