Myeloma Patients Europe

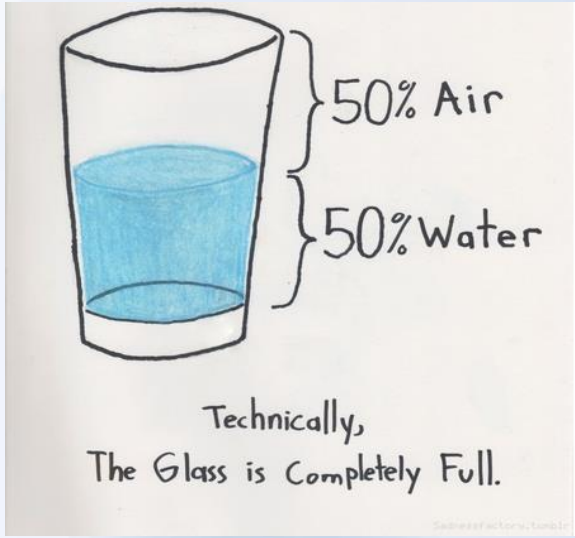
EMPOWERING MYELOMA ADVOCACY ACROSS EUROPE

Statistics for dummies

and for patient advocates ☺

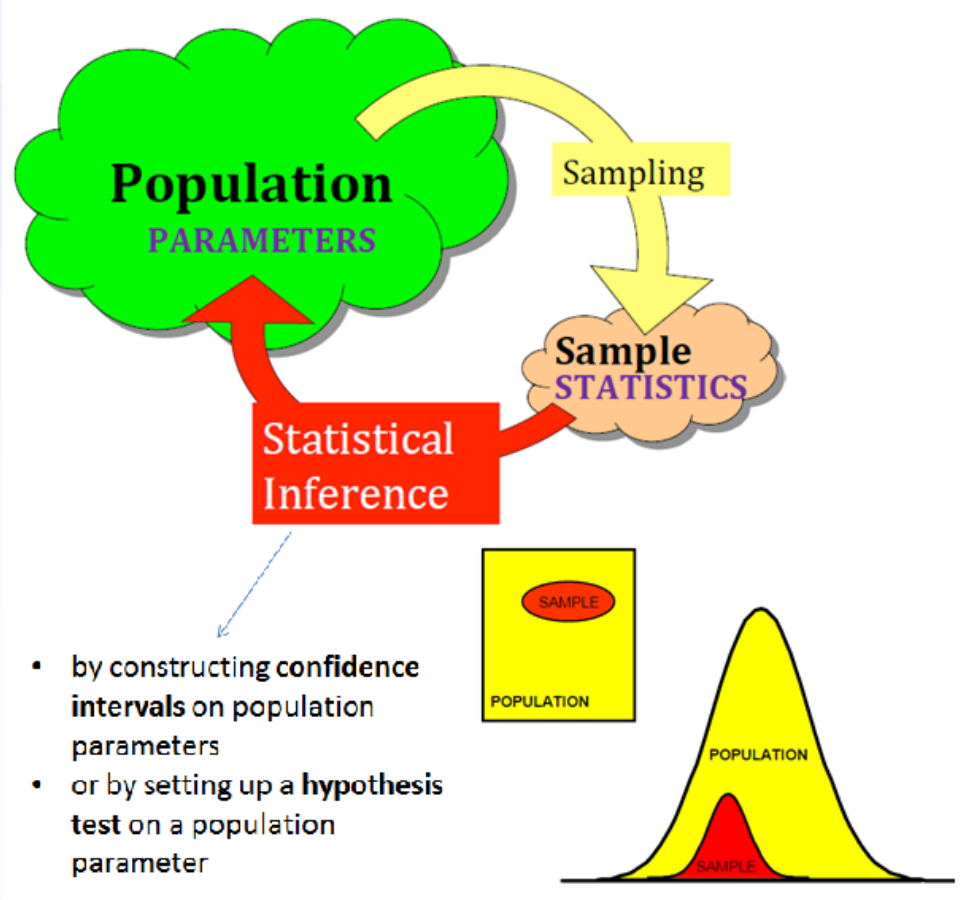
What is statistics and why do we need it?

- The „science of the state" – originally developed to assess the wealth and power of states
- Scientific thinking and method: exact and objective measurement (as much as possible)
- The science of **collecting**, **organising** and **interpreting** data
- **Statistics is also a matter of interpretation**



Types of statistics

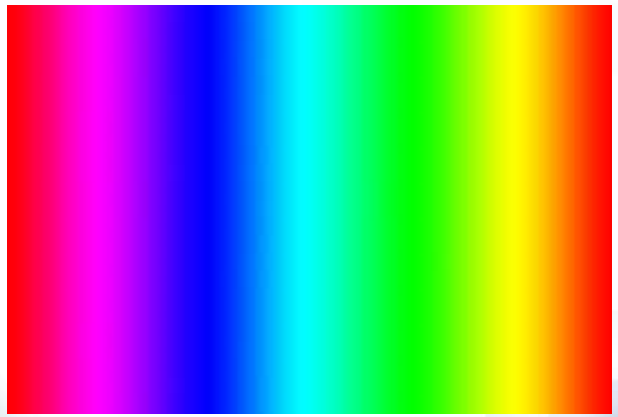
- **Descriptive statistics** – Methods of organizing, summarizing, and presenting data in an informative way
- **Inferential statistics** – The methods used to determine something about a population on the basis of a sample
  - Population –The entire set of individuals or objects of interest or the measurements obtained from all individuals or objects of interest
  - Sample – A portion, or part, of the population of interest

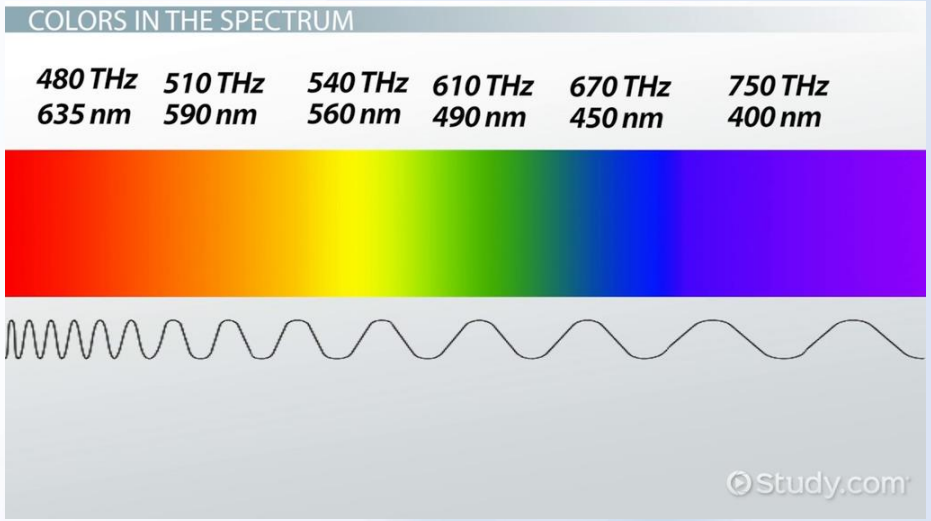


https://mahritaharahap.wordpress.com/teaching-areas/inferential-statistics/

What are data?

- Information in raw or unorganized form (such as alphabets, numbers, or symbols) that refer to, or represent, conditions, ideas, or objects.
- Data can be in many forms
  - Continuous / discreet
  - Analog / digital
  - Quantitative / qualitative



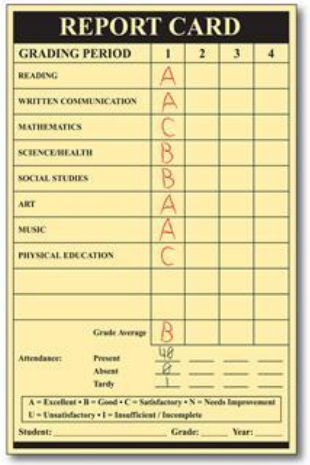


https://study.com/academy/lesson/the-nature-of-light-origin-spectrum-color-frequency.html

Read more: http://www.businessdictionary.com/definition/data.html

Types of data

- Continuous
  - Equal increments
- Ordinal/Rank
  - In order but not equal (Likert)
- Categorical
  - Names



http://web.engr.oregonstate.edu/~burnett/CS589empirical/CS589-statisticalStudies/Rstuff/Jen-resources/Statistics%20Made%20Easy.ppt

Collecting data

- **Systematic** gathering and ordering of data
- Many different methods:
  - Survey
  - Experiment
  - Observation

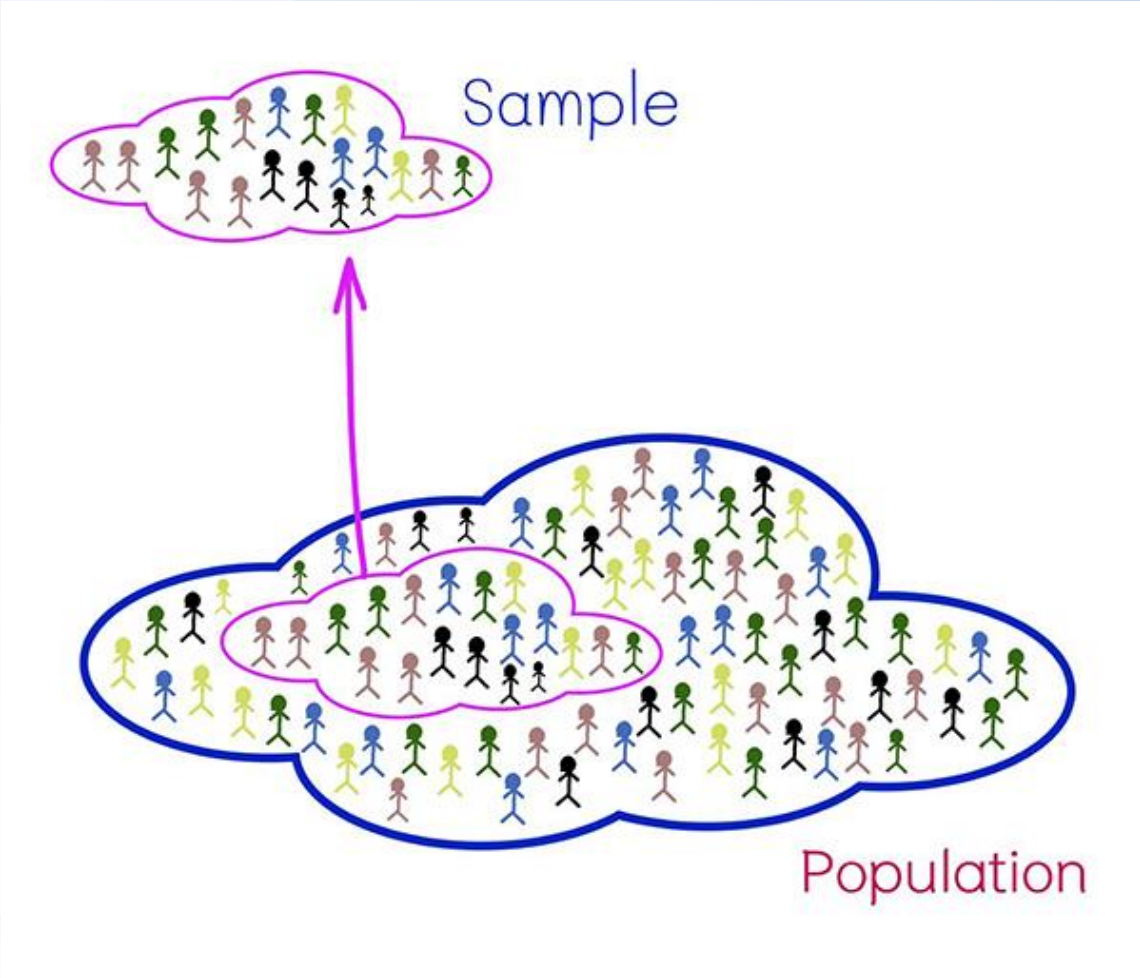
- The scientific method of data collection tries to ensure that data are consistent and systematically organised for comparison and analysis
- **We do this to answer a QUESTION! (so there should be a question)**

Sampling

Sampling methods can be:

- **Random** (each member of the population has an equal chance of being selected**)**
- **Non-random**

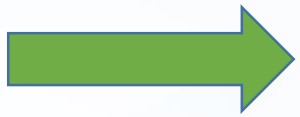
- The actual process of sampling causes **sampling errors**. For example, the sample may not be large enough or representative of the population. Factors not related to the sampling process cause non-sampling errors. A defective counting device can cause a non-sampling error.



What is a question?

- **Statistical question:** a question where you expect to get a variety of answers, and you are interested in the distribution and tendency of those answers.

How are you today?

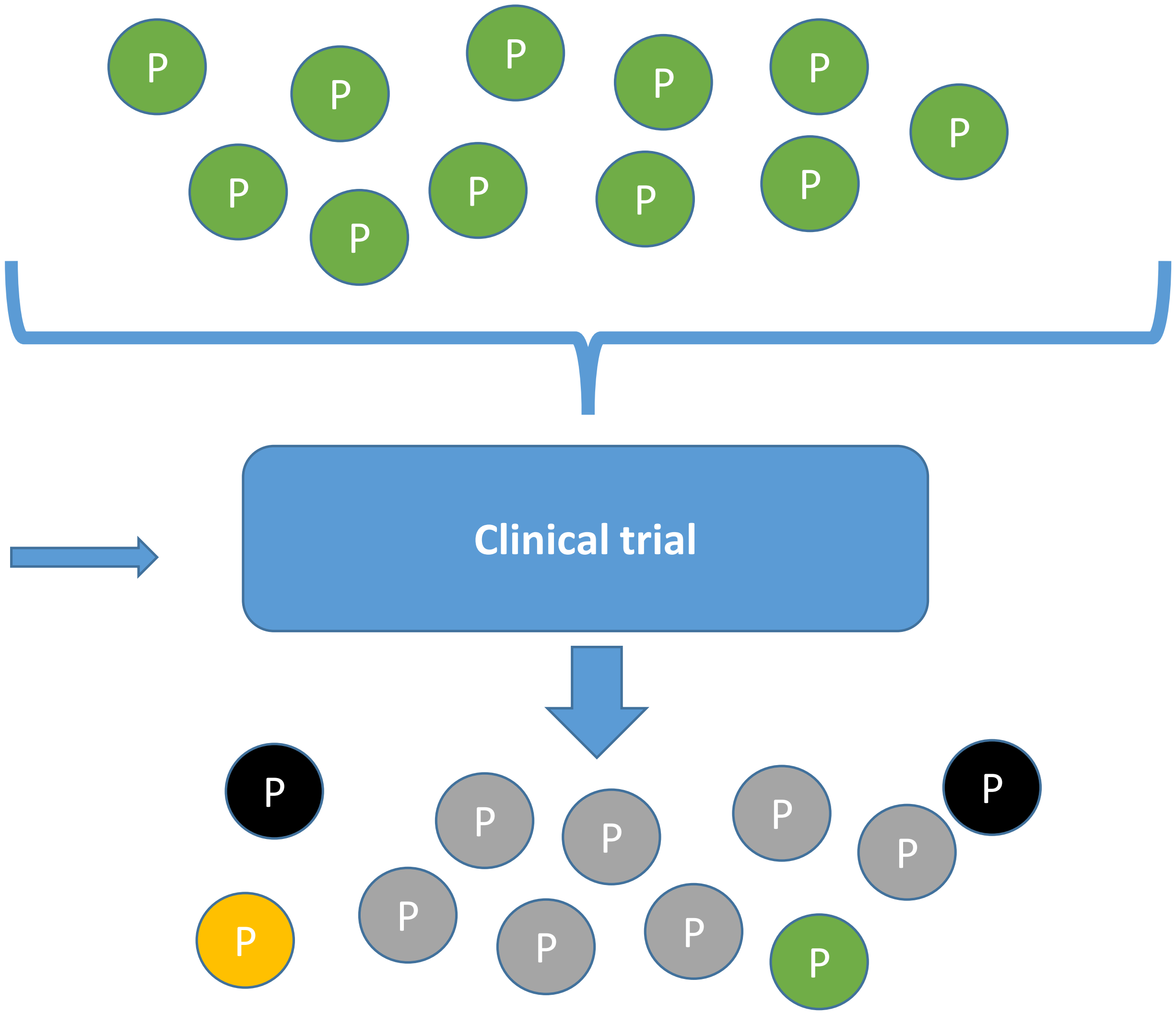


Please rate your mood today from 0 to 10

- **Quantifiable answers.** Maybe not always. Then coding is used.

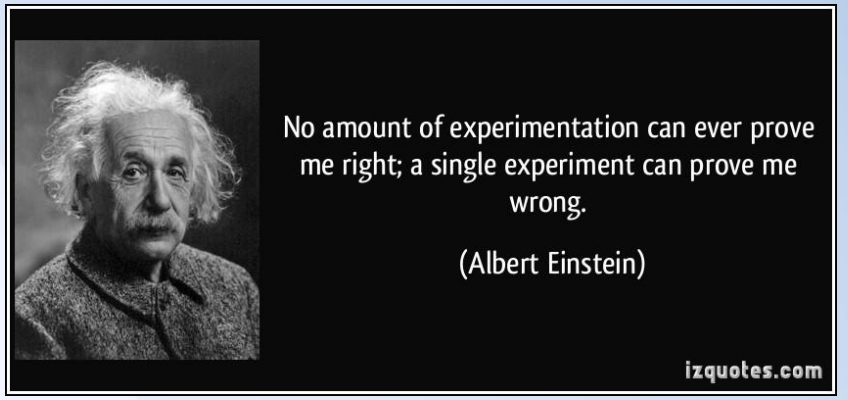
A clinical trial is an experiment

What's going on here?



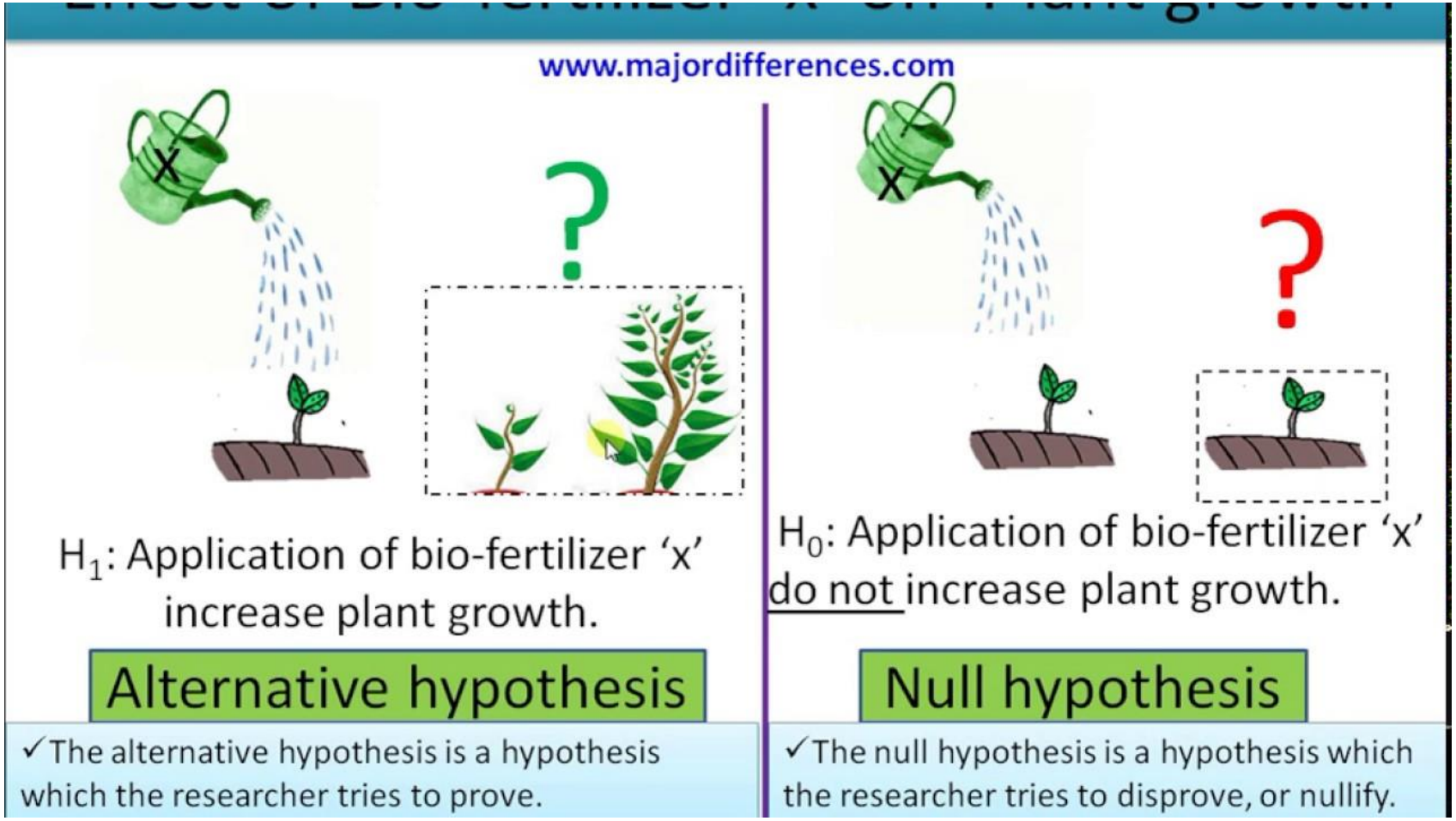
Measurement
Statistics

Null hypothesis



- Hypothesis = assumption, or set of assumptions, that either a) asserts something on a provisional basis with a view to guiding scientific investigation; or b) confirms something as highly probable in light of established facts.
- Rather than trying to prove the B hypothesis, scientific method assumes that in fact A is true – that there is no difference between the standard of care and the new treatment. This is known as the 'Null' hypothesis. The scientists then try to disprove A.
- The null hypothesis covers our current situation or knowledge), which we need to trust unless we have sufficient evidence otherwise. But if we sought to prove the 'alternative hypothesis' (as it is known, opposite the 'null hypothesis') then in effect 'the accused is guilty'.
- Trying to prove the null hypothesis false or wrong is a more rigorous, and achievable objective than trying to prove the alternative hypothesis is right.

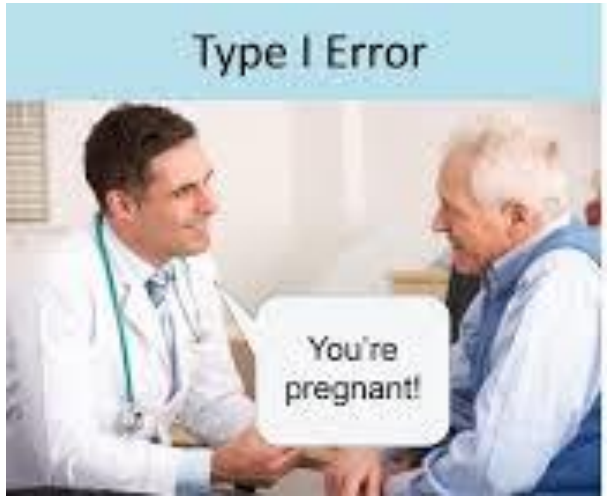
Null hypothesis



https://www.majordifferences.com/2016/10/5-differences-between-null-and.html

Type I and type ii errors

		The Truth (Based on Entire Population)	
		Nothing Is There (H_0 Is True)	Something Is There (H_0 Is False)
Your Conclusion (Based on Your Sample)	I Don't See Anything (Nonsignificant)	Right!	Wrong (Type II Error)
	I See Something (Significant)	Wrong (Type I Error)	Right!



NORMAL DISTRIBUTION

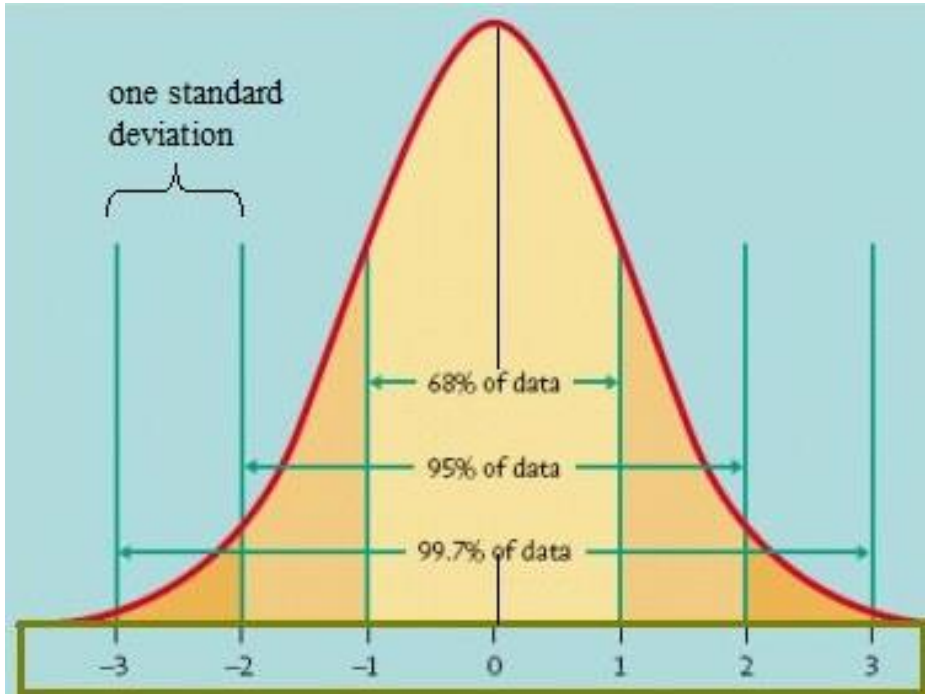
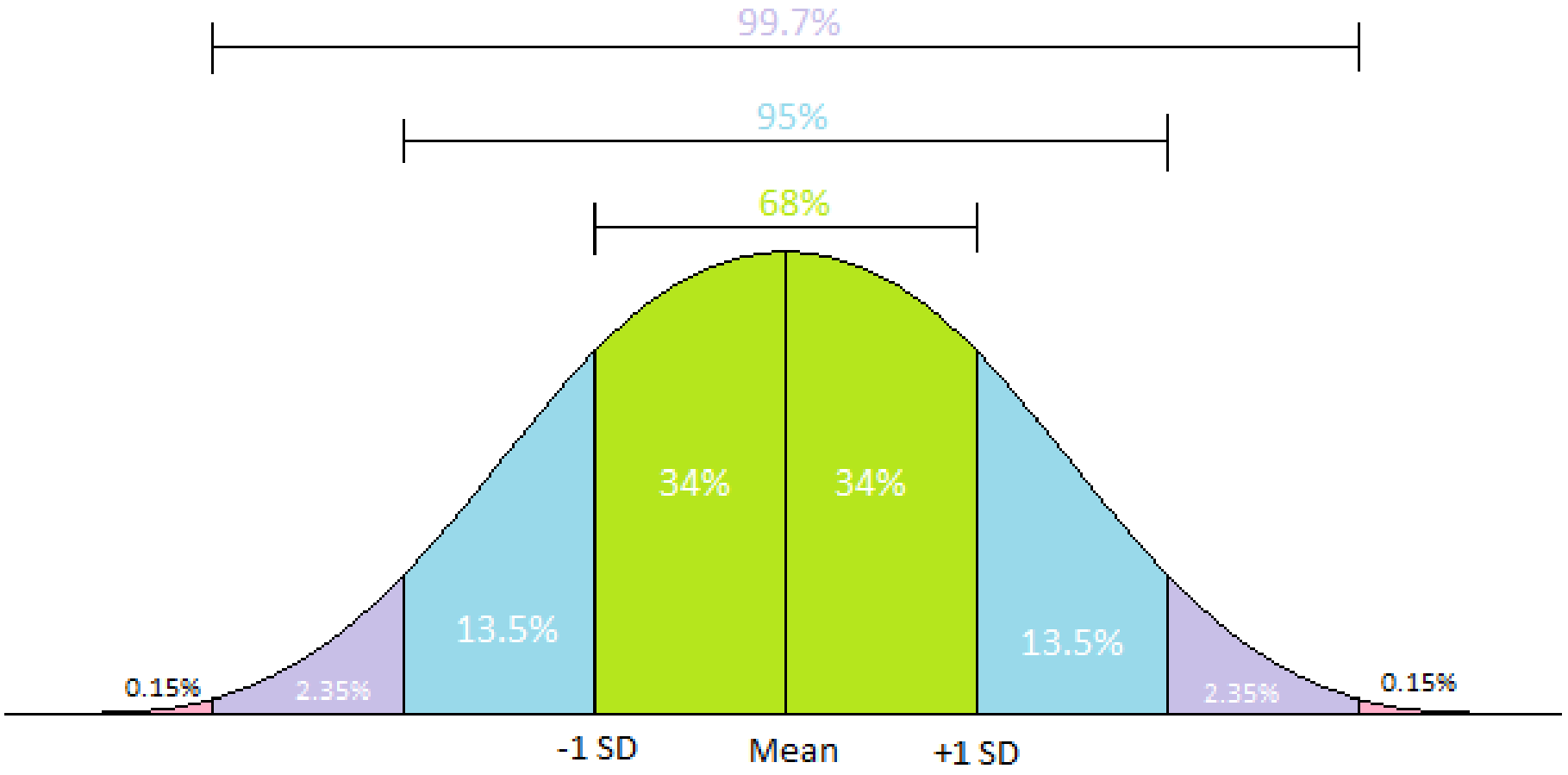
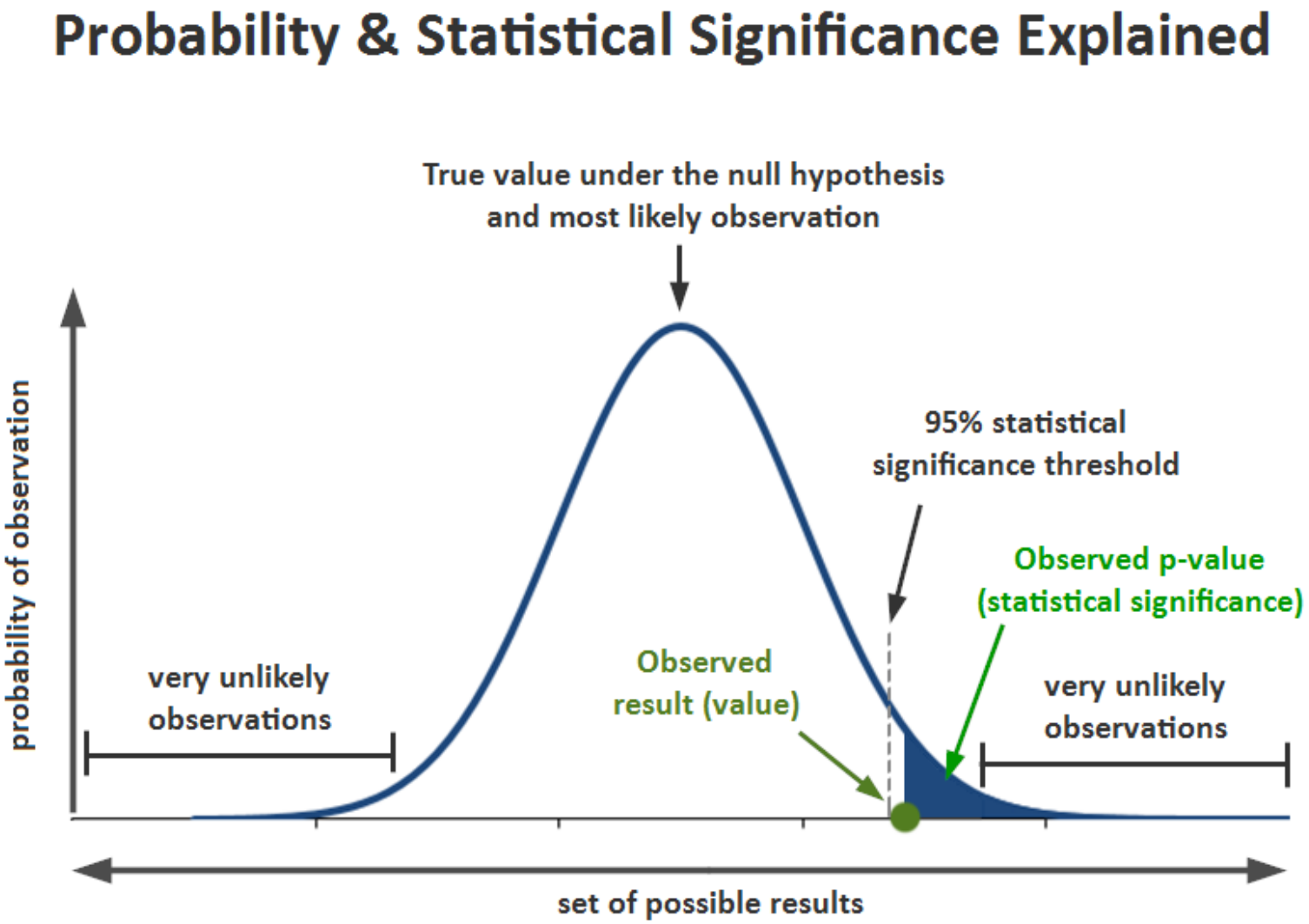


Photo courtesy of Judy Davidson, DNP, RN

Standard Deviation (σ)

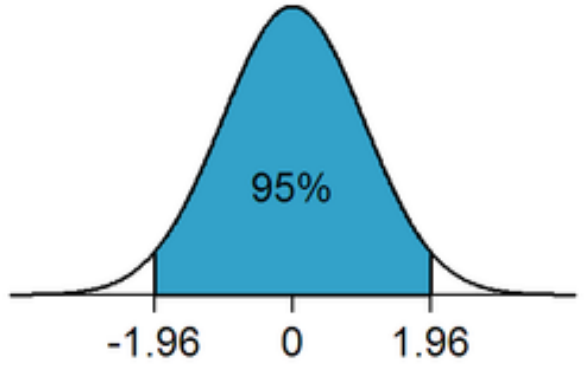


Statistical significance



http://blog.analytics-toolkit.com/2017/statistical-significance-ab-testing-complete-guide/

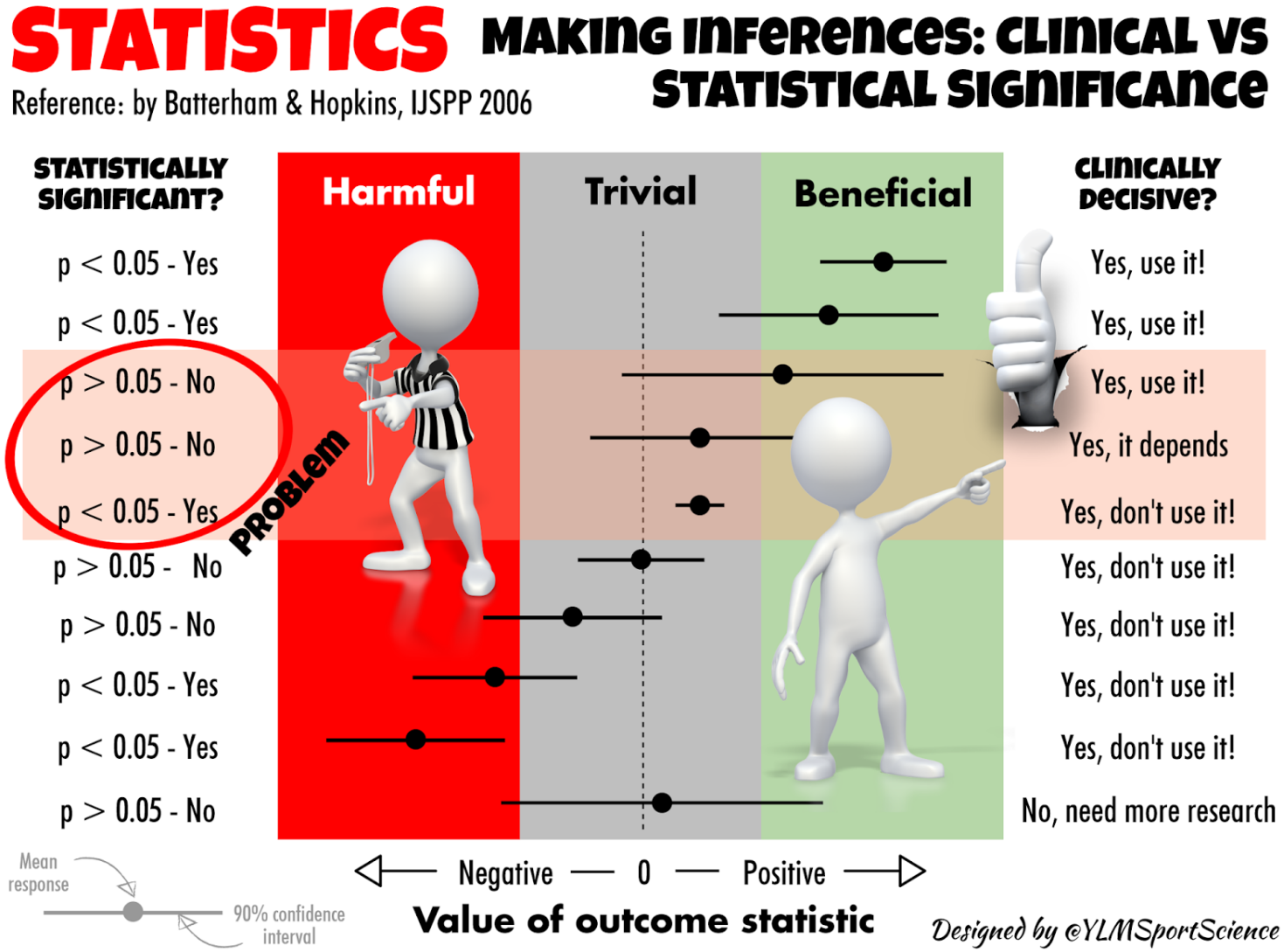
We have a result. But is it significant? And how confident can we be that it is?



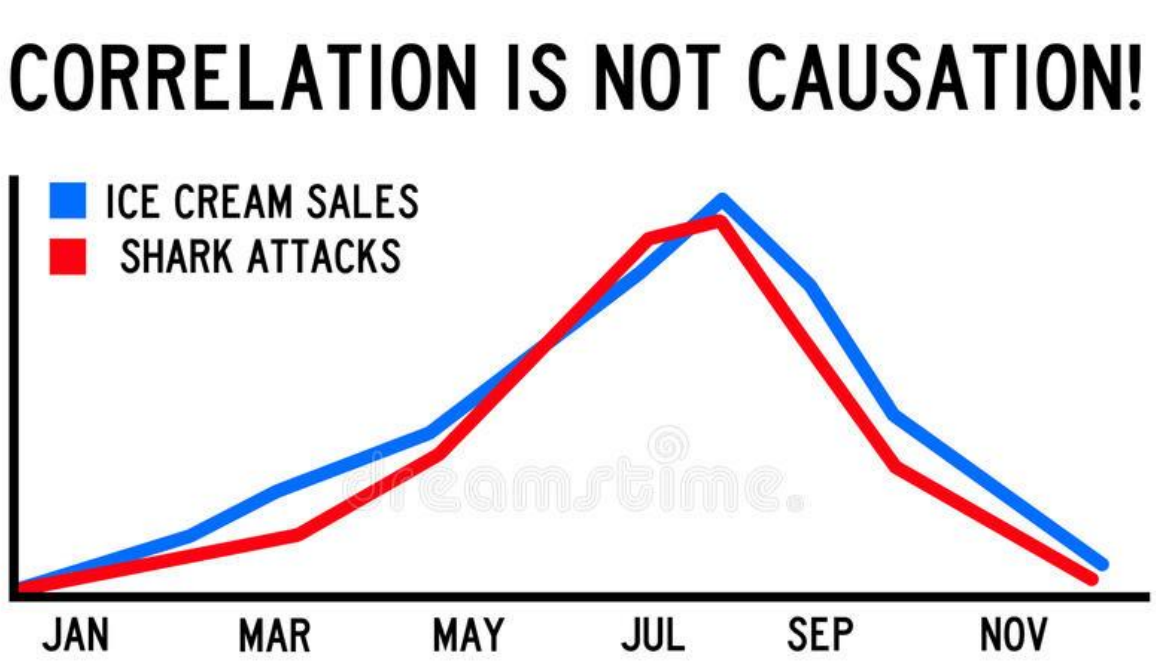
The *p* value tells us.
Arbitrary but useful.
Tells if accidental or real finding.

Clinical relevance

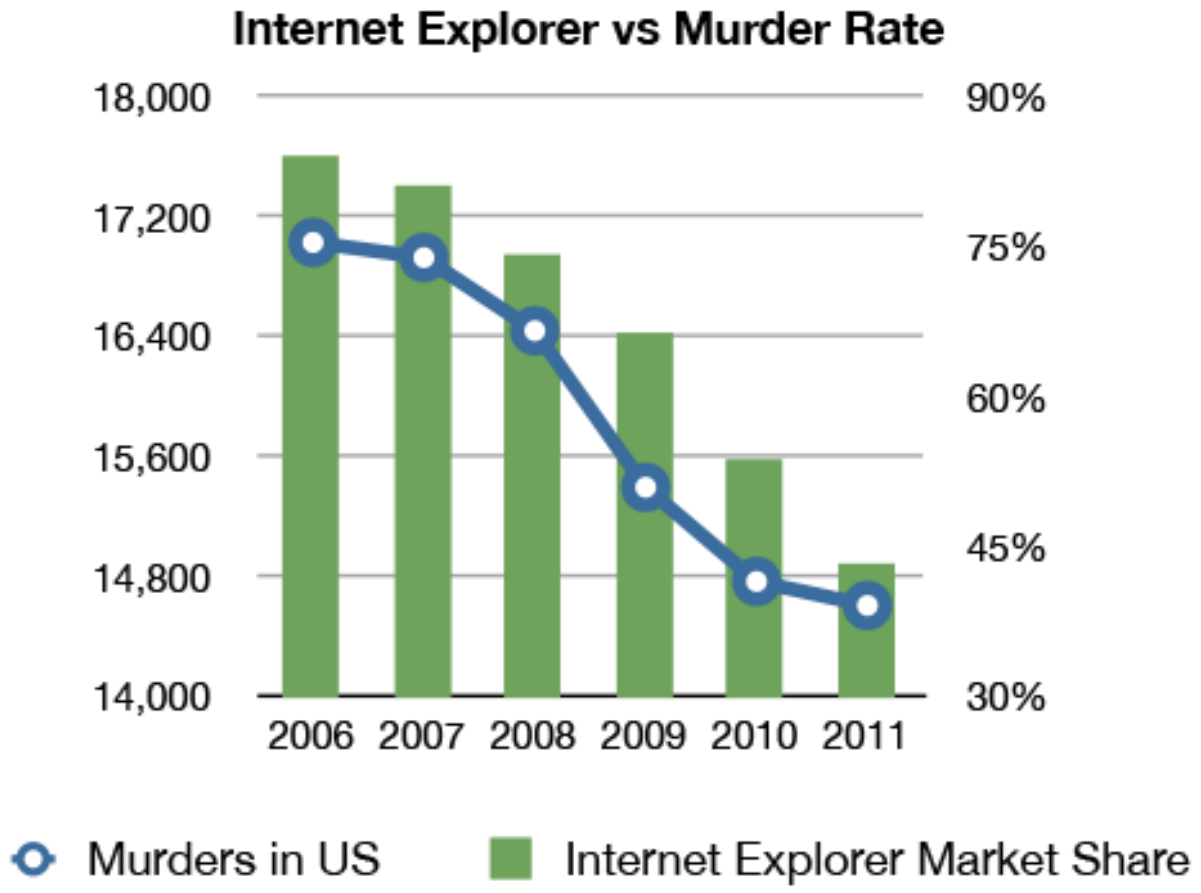
We have a result. Is it clinically relevant? Can we use it for anything?



Correlation or causation?



Both ice cream sales and shark attacks increase when the weather is hot and sunny, but they are not caused by each other (they are caused by good weather, with lots of people at the beach, both eating ice cream and having a swim in the sea)



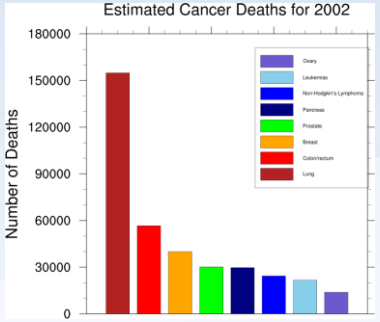
Some more advanced concepts

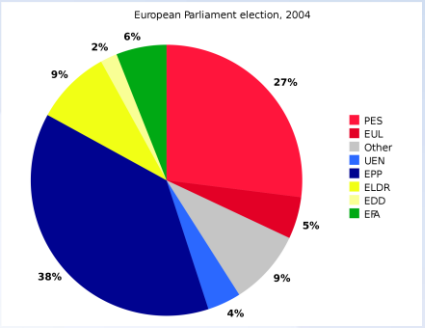
- Bother with tables?
- Reading charts
- Outliers
- Kaplan-Meier curves
- Hazard ratios

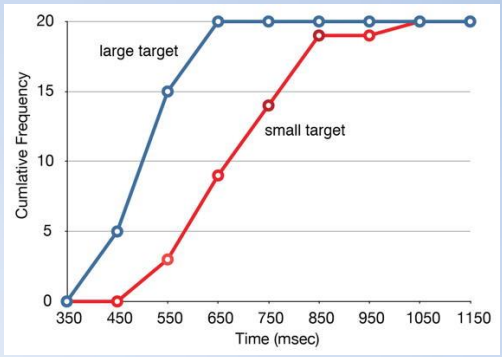
Tables

- There’s an app for that! – Use technology like Excel or SPSS and other stats packages
- Look at the analysis and the summary
- Keep tables at hand and check back for data

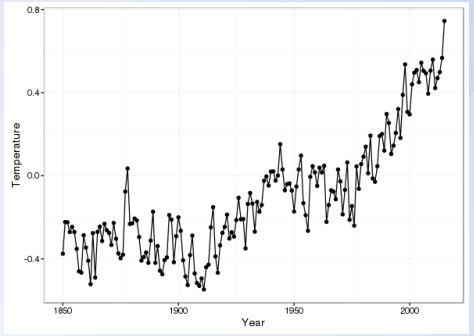
Charts

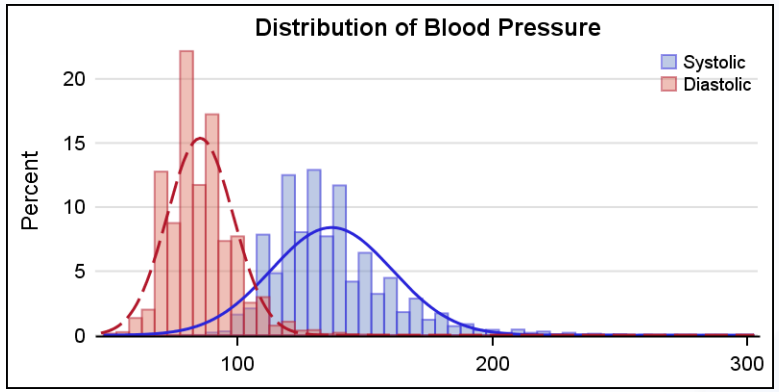


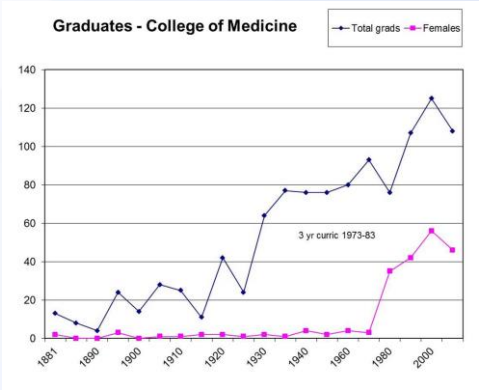




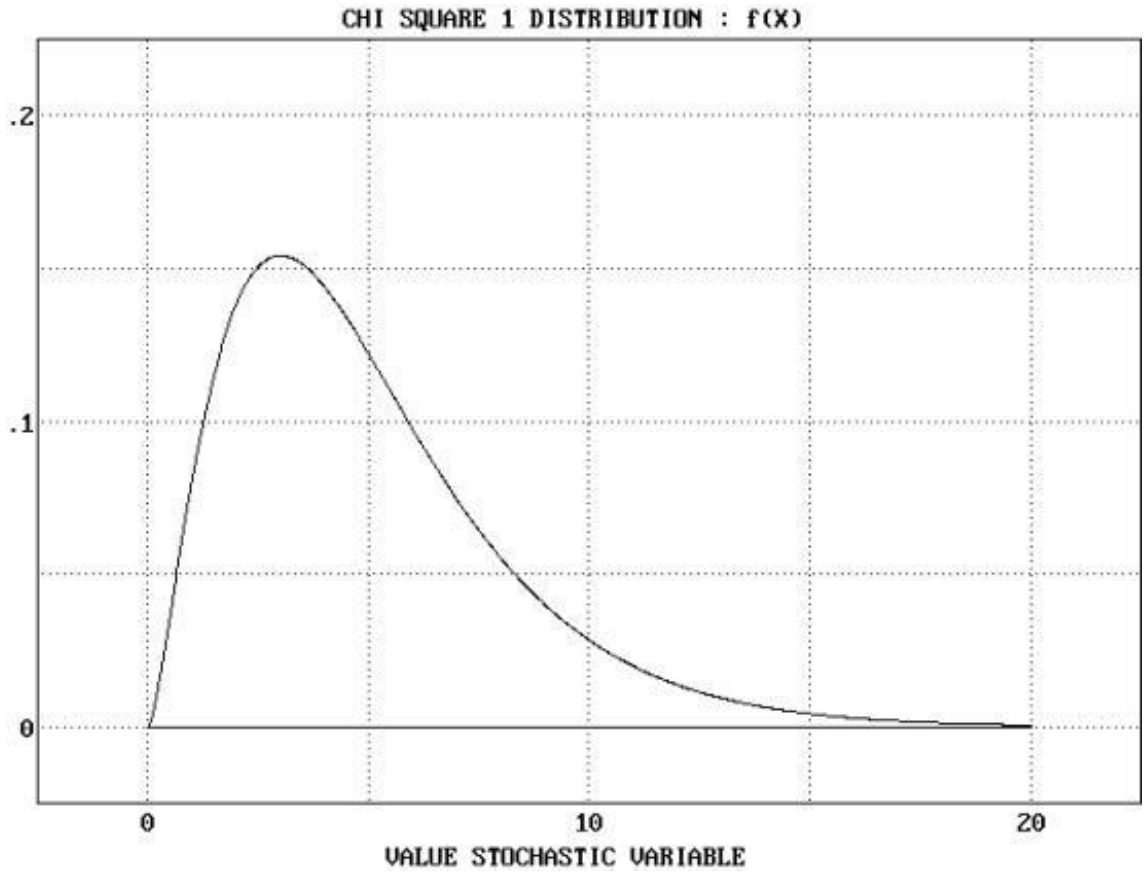
- Frequency distributions are good ways to present the essential aspects of data collections in concise and understable terms
- Pictures are always more effective in displaying large data collections
  - Histogram
  - Frequency polygon
  - Ogive
  - Pie chart
  - Bar chart
  - Time series
  - Kaplan-Meier curve
  - Etc.





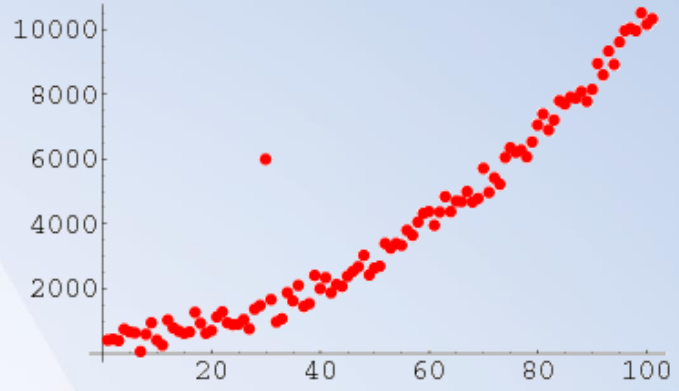


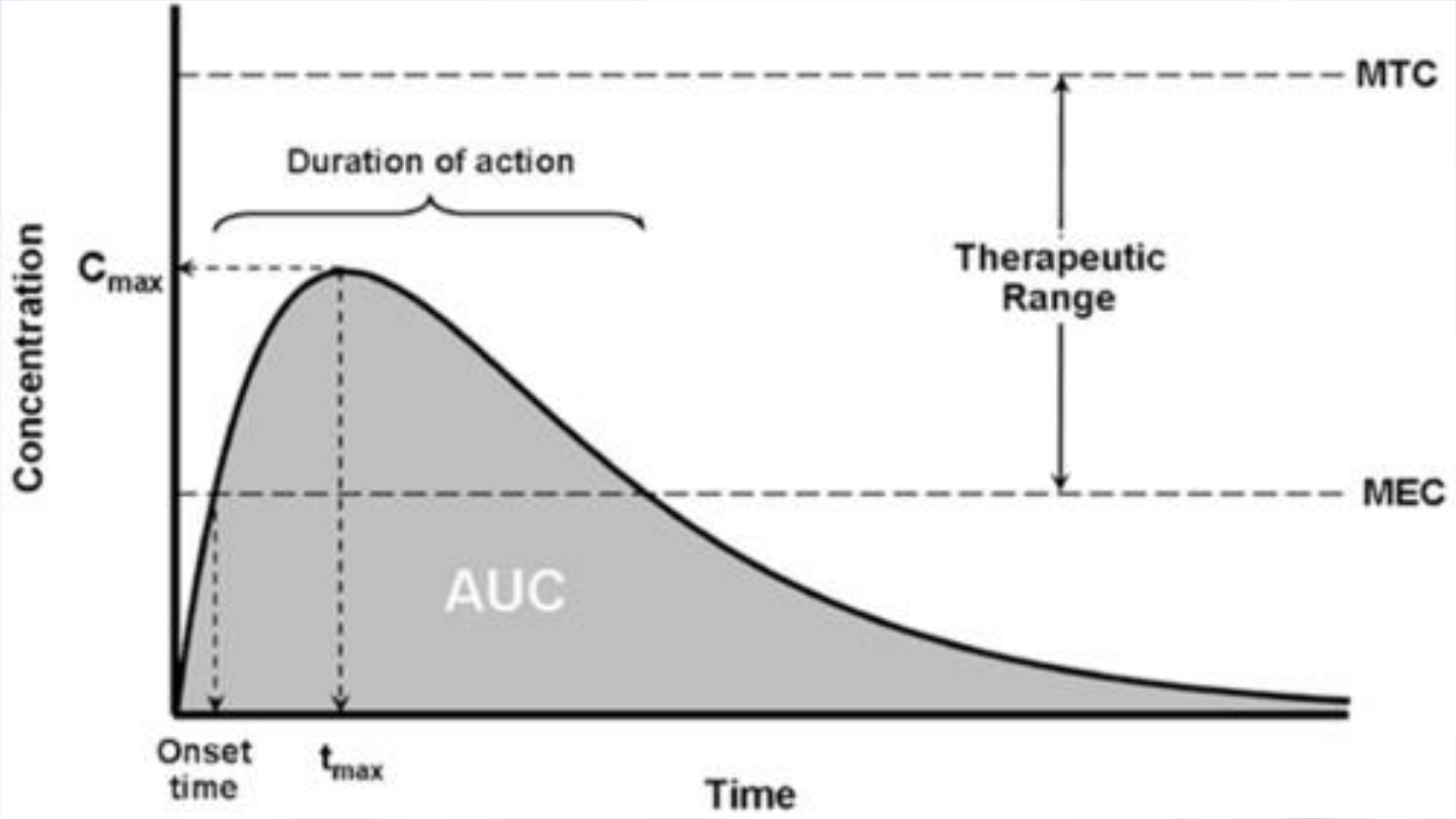
Chi-Square (χ^2)



- Any number squared is a positive number
- Therefore, area under the curve starts at 0 and goes to infinity
- To be statistically significant, needs to be in the upper 5% ($\alpha = .05$)
- Compares observed frequency to what we expected

Outliers and AUC

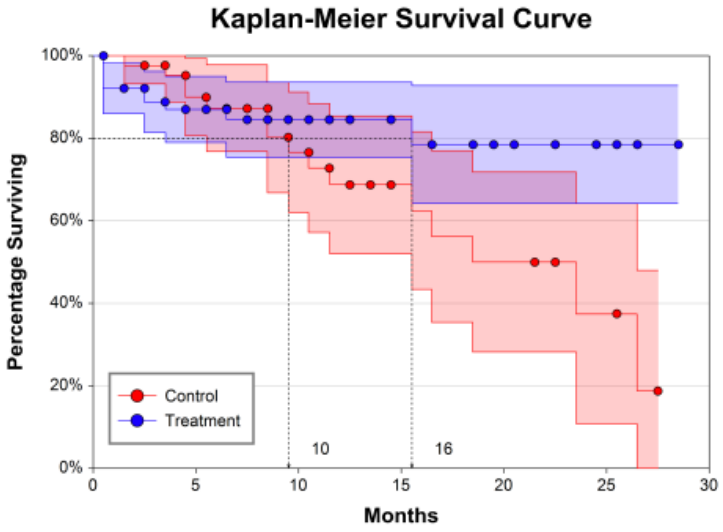


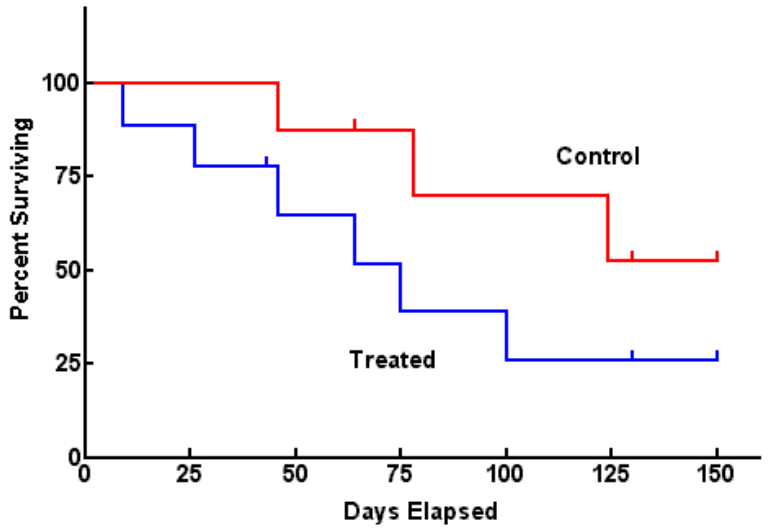


Look at the area under the curve
But also look at the outliers!

https://www.rhealth.com/rhealth-partnering/

Kaplan-Meier Curve





- Length of time from study entry to disease end-point for a treatment and control group
- From this curve, we can derive:
  - median time (time at which 50% of cases resolve)
  - mean time (average resolution time)
- Allows comparisons of patients throughout study and provides information on patients who may be lost to follow up
- Can also be used in other fields like engineering (Kaplan's original work on vacuum tubes)

- Edward Kaplan and Paul Meier discovered in 1958, independently from each other, then worked together on a paper
- Estimating patient survival rates on the basis of both patients in the trial and dropped out from the trial (censored)
- Taking into account the fact that some patients may have died during a research trial while others will survive beyond the end of the trial
- Using information from those who have died and those who have survived to estimate the proportion of patients alive at any point during the trial
- When a large enough sample is taken, approaches the true survival function for that population

Hazard ratios

$$Hazard\ ratio = \frac{hazard\ in\ exposed\ group}{hazard\ in\ unexposed\ group}$$

- A hazard rate is the rate at which a particular event happens
- The hazard ratio = treatment hazard rate/placebo hazard rate, i.e. the ratio of the particular event taking place in treatment group compared to control group
- Need to interpret hazard ratio alongside a measure of time
- Used to reflect time survived to an event
- Does not indicate how fast something occurs
- Commonly used when presenting data from a clinical trial (not the same as a relative risk ratio)
- Quantifies ‘the odds of winning the race’ not the margin of victory (see median ratio)
- Hazard ratio of 1 = equal event rate between groups
- Hazard ratio of 2 = twice as many patients in the active group will have the event compared to the control in the next unit of time
- Hazard ratio of 0.5 = half as many patients in the active group are having the event compared to the control in the next unit of time

Additional resources

- Bettina Ryll's webinar on reading science: https://youtu.be/H_V0tUBjBBM
- Crash course statistics on YouTube: https://youtu.be/zouPoc49xbk?list=PL8dPuuaLjXtNM_Y-bUAhblSAdWRnmBUcr
- Basics of statistics course book: https://www.mv.helsinki.fi/home/jmisotal/BoS.pdf