Statistical considerations when planning your research project

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Resources for statistical assistance

Department of Statistics at UBC:

www.stat.ubc.ca/how-can-you-get-help-your-data

SOS Program - An hour of free consulting to UBC graduate students. Funded by the Provost and VP Research Office.

STAT 551 - Stat grad students taking this course offer free statistical advice. Fall semester every academic year.

Short Term Consulting Service - Advice from Stat grad students. Fee-for-service on small projects (less than 15 hours).

Hourly Projects - ASDa professional staff. Fee-for-service consulting.

Hands-on workshops in R: `http://ecoscope.ubc.ca/events/`

Outline

Types of research studies

Planning your research study

Sample size calculations

Experimental design

Multiple comparisons

Types of analysis

Types of research studies

Sample Surveys

- Information about a population

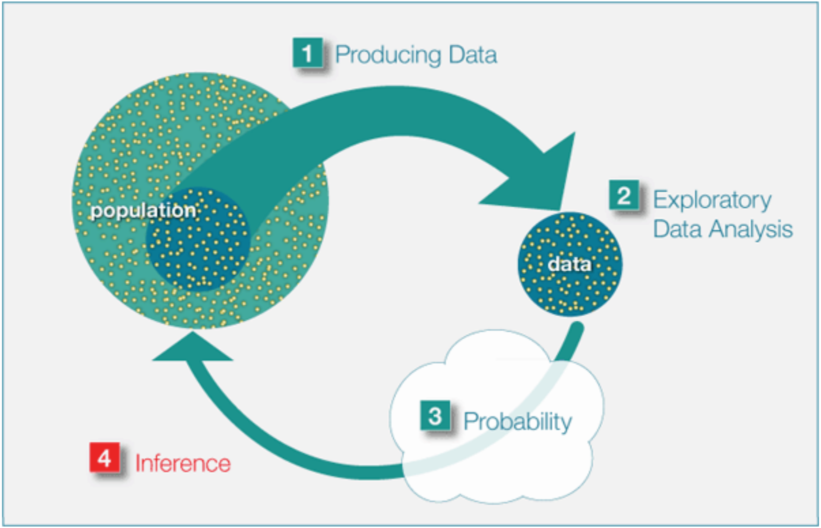
Observational Studies

- Interested in comparing two or more groups
- Assignment of groups is not controlled

Controlled Experiments

- Interested in comparing two or more groups
- Group membership is assigned randomly

The Population and The Sample



Sampling Error

A Sample is not a perfect representation of the population:

- Take one sample, get one answer
- Take another sample, get a different answer

Rasmussen US Election Poll

1500 people surveyed online and by phone two weeks before the 8th November 2016 (last US presidential elections)

Estimated percentages from the sample: 45% Clinton, 42% Trump

Margin of Error: 42% - 48% Clinton, 39% - 45% Trump

Sample Surveys

Focus on certain characteristics of interest

Target Population of size N (usually finite)

Random sample of size n from the population

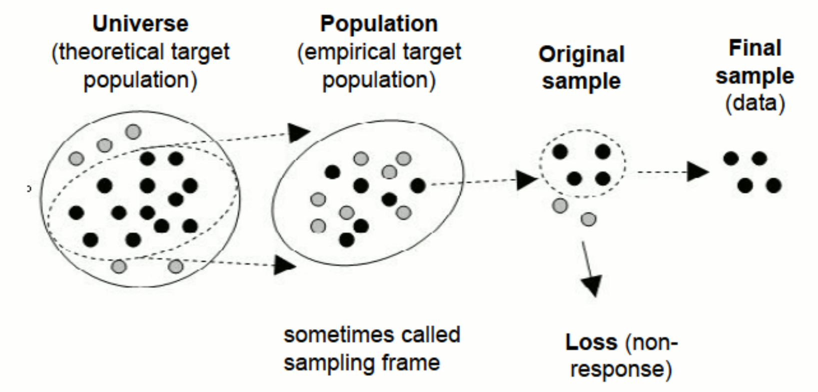
- yields data $x_1, \ldots x_n$

Use data from the random sample to make inference about the characteristics of interest

- compute sample (point) estimates
- compute error associated with those estimate
- compute interval estimates with desired probability coverage

sampling fraction could be important n/N

From Target Population to Final Sample



Roosevelt vs Landon: 1936

Election Poll by Literary Digest during the Great Depression

Sample of 10 million from their readers, telephone users, and automobile owners

n = 2.4 million responded

Poll result:

- 57% for Landon, 36% for Roosevelt

Actual Election Result:

- 36.5% for Landon, 60.8% for Roosevelt

Discussion point: Why do you think Literary Digest was so inaccurate?

Issues with Sample Surveys

Establishing the sampling frame

Over-coverage or Under-coverage:

- Is the entire population in the sampling frame?
- Are subjects outside the population in the sampling frame?

Selection bias

- is the sample representative of the population?

Non-response bias

- Do non-responders differ from responders?

Wording and order of the questions

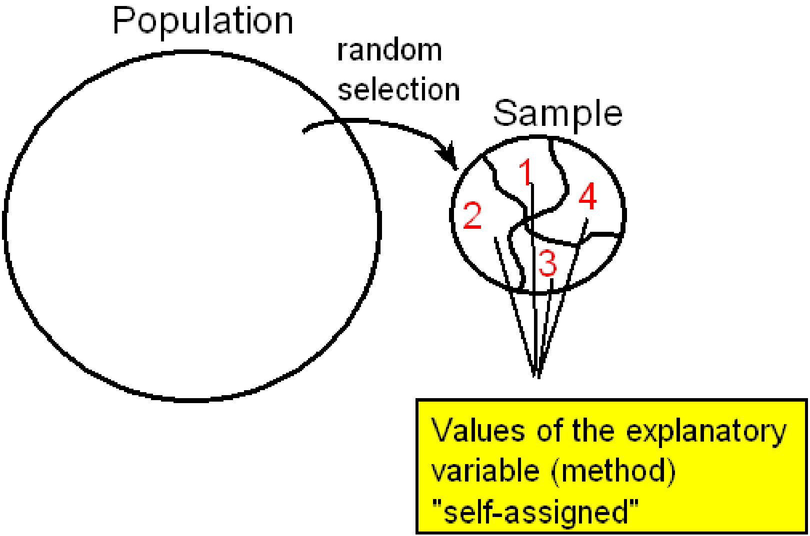
Brexit

Supporters of the trailing opinion are less likely to respond to surveys, biasing the result in favor of the more popular opinion

As the climate changes, the swings may be over exaggerated

`http://uk.businessinsider.com/pollsters-know-why-they-were-wrong-about-brexit-2016-7`

Observational Studies



Observational Studies

Characteristics of interest compared between 2 or more distinct populations

- male vs female, exposed vs unexposed

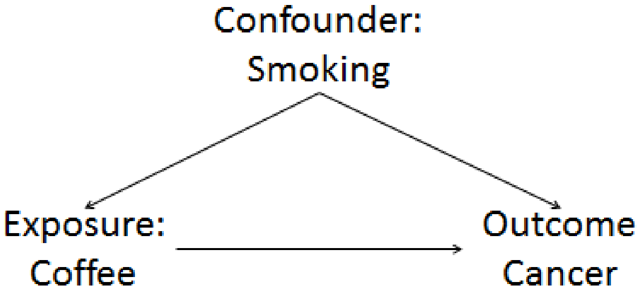
Random sample from each population

- often based on who's available

Use data to compare the characteristics of interest between populations

- compute sample estimates and their errors
- compare the sample estimates

Observational Studies



Confounding

This is a real-life example from a medical study comparing the success rates of two treatments for kidney stones. The table below shows the success rates and numbers of treatments for treatments involving both small and large kidney stones, where Treatment A includes all open surgical procedures and Treatment B is percutaneous nephrolithotomy (**Wikipedia**)

Size	Treatment A	Treatment B
Small Stones	**93% (81/87)**	87% (234/270)
Large Stones	**73% (192/263)**	69% (55/80)
Combined	78% (273/350)	**83% (289/350)**

If confounding variable of severity of condition is taken into account the conclusions are reversed (Simpson's paradox or the Yule–Simpson effect).

Issues with Observational Studies

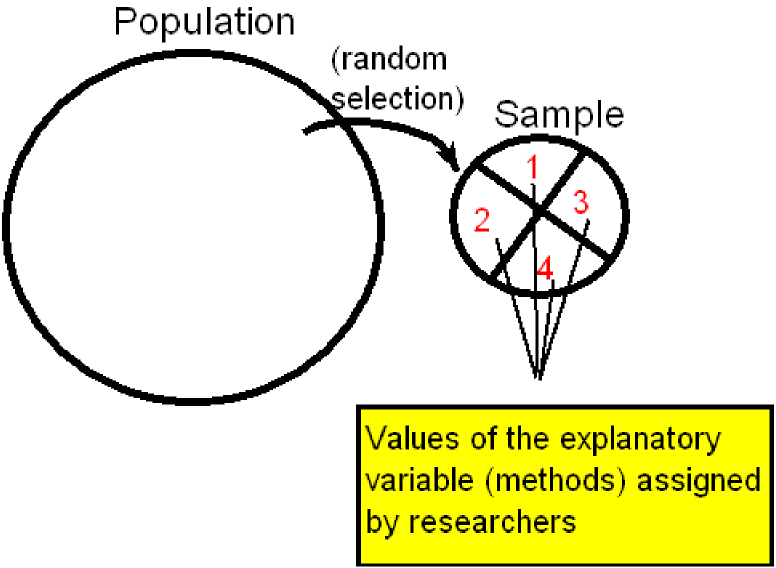
Confounding is a major issue

- Differences could be due to another factor that differs across the groups
- Analyses must adjust for such confounders
- Extreme cases can lead to Simpsons paradox

Analyses can suggest, but not establish causality

- Bradford Hill's criteria for causality

Controlled Experiments



Controlled Experiments

Characteristics of interest compared between 2 or more groups

Random sample is drawn from the study population

- sometimes from several populations if some observational factors are to be controlled (i.e. smokers and non-smokers, high severity and low severity)

Subjects are randomized to groups and data is collected

- must be able to control the group assignment

Use data to compare the characteristics of interest between groups

- compute sample estimates and their errors
- compare the sample estimates

Aspects of Controlled Experiments

It has been observed that patient's belief does indeed sometimes result in therapeutic effect and cause patient's condition to improve

- use a placebo, a sham treatment to remove this effect
- subjects are not aware of which group they have been placed

It is very easy for a researcher, even subconsciously, to influence experimental observations

- use double blind experiment where both researchers and subjects are unaware of the group assignments

The discipline of Statistics

Wikipedia: Statistics is the study of the collection, organization, and interpretation of data

American Heritage Science Dictionary: The branch of mathematics that deals with the collection, organization, analysis, and interpretation of numerical data

Merriam-Webster Dictionary: A branch of mathematics dealing with the collection, analysis, interpretation, and presentation of masses of numerical data

Dictionary.com: the science that deals with the collection, classification, analysis, and interpretation of numerical facts or data, . . .

Planning your Study

Before you collect any data

Define the questions of interest

Determine the appropriate populations that will allow the questions to be answered

Create a plan to sample the populations and a randomization scheme if required

Determine what information (data) is needed from the sample to answer the questions

Create an appropriate analysis plan

Determine the sample size needed

Defining the Question

What do you want to know?

Make sure questions are clear and focused

All questions should be based on the same populations or perhaps a subset

Each question should define a single hypothesis to test or quantity to measure

The Population

Who or what are you going to study?

Can you establish a proper sampling frame? Does it cover the population of interest?

If the sampling frame is a subset of the population of interest and it varies systematically from the population, the results cannot be generalized

Observational studies usually compare more than 1 self selecting population

The Sampling Plan

How should you select units from the population?

For surveys a poor sample means unreliable results

Experiments and observational studies usually rely on who is available

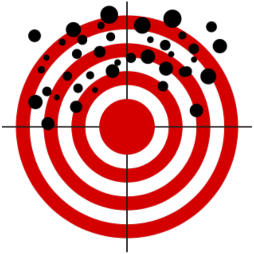
Does the sample represent the population?

- This is who is sampled not how many
- Also known as accuracy or bias

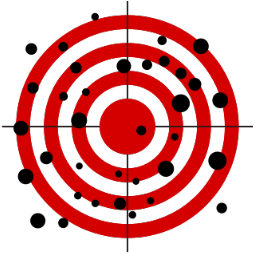
Does the sample give enough precision?

- This is how many are sampled
- Also known as power in testing

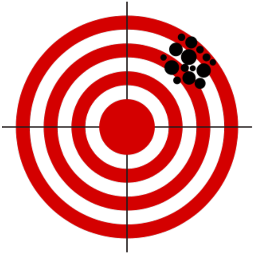
Accuracy vs Precision



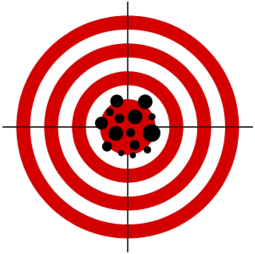
Unreliable & Unvalid



Unreliable, But Valid



Reliable, Not Valid



Both Reliable & Valid

Randomization

```
##   Subject Treatment Assignment
## 1 - Dave         A          4
## 2 - Pete         A          3
## 3 - Josh         A          5
## 4 - Elly         B          2
## 5 - Suzy         B          1
## 6 - John         B          6
```

Restricted Randomization

Randomization done separately within Male and Female

```
##   Subject Treatment Assignment
##  1 - Elly         A          2
##  2 - Suzy         B          1

##
##  1 - Dave         A          3
##  2 - Pete         A          2
##  3 - Josh         B          1
##  4 - John         B          4
```

Randomization

For unit selection and allocation to groups

Relevant for surveys, observational studies and controlled experiments

Eliminates bias from the study by allowing:

- sampled units to represent the population, won't favor a particular type of group
- unobserved confounders to be distributed randomly between groups

Differences can still occur by chance

Randomization can be done within the levels of observational factors, i.e. size of kidney stone, severity of disease

The Data

What information is needed to answer your questions?

Is the data collection procedure reliable?

Is the data entry accurate?

Are the data complete?

Can you confirm or correct suspected problems in the data?

Can you fill in missing data after the fact?

The data issues should be resolved before analysis begins

The Analysis Plan

How you answer your questions

Should include graphics to examine the data

Analysis is mainly determined by the type of dependent/response variable

- continuous, count, binary, categorical

Hypothesis testing or parameter estimation

Are the data truly independent?

- repeated measures or cluster effects

Do you need to adjust for any confounders?

Do you have a multiple comparison problem?

The Effects of Sample Size

None on accuracy, only precision

1. Sampling distribution of the means when population follows normal distribution

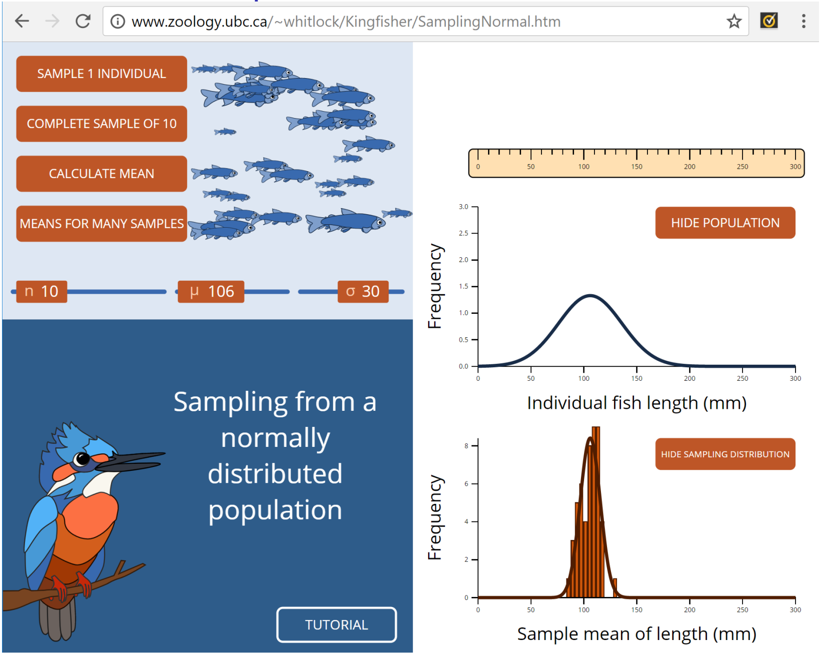
http://www.zoology.ubc.ca/~whitlock/Kingfisher/SamplingNormal.htm

2. Sampling distribution of the means when population follows non-normal distribution

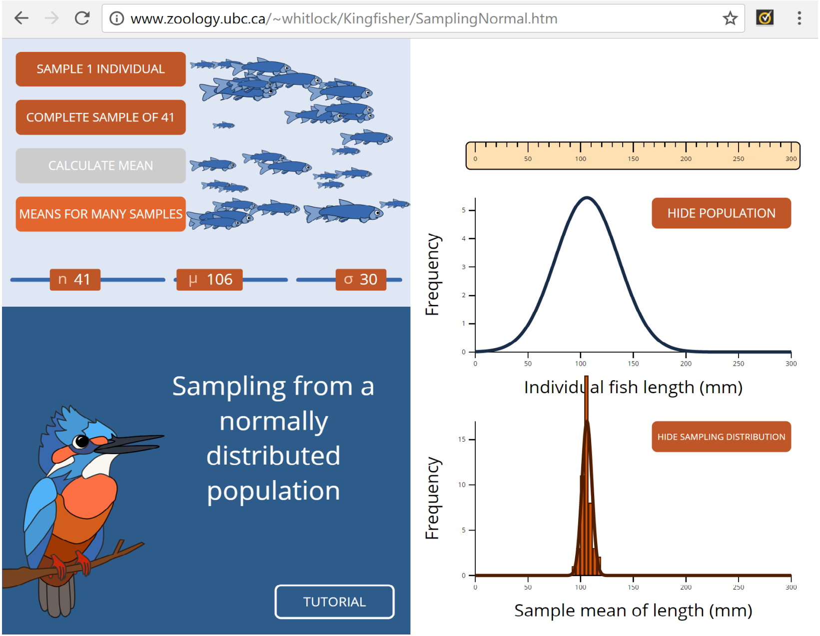
http://www.zoology.ubc.ca/~whitlock/Kingfisher/CLT.htm

For more stat tutorials visit
https://statspace.elearning.ubc.ca/

The Effects of Sample Size



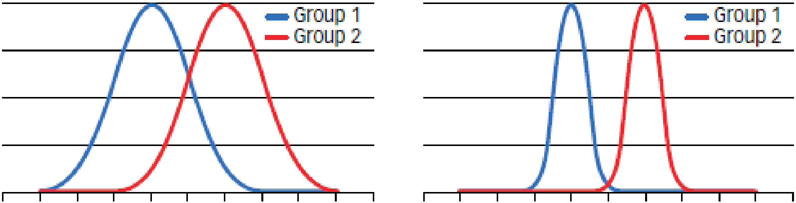
The Effects of Sample Size - cont



The Effects of Sample Size - cont

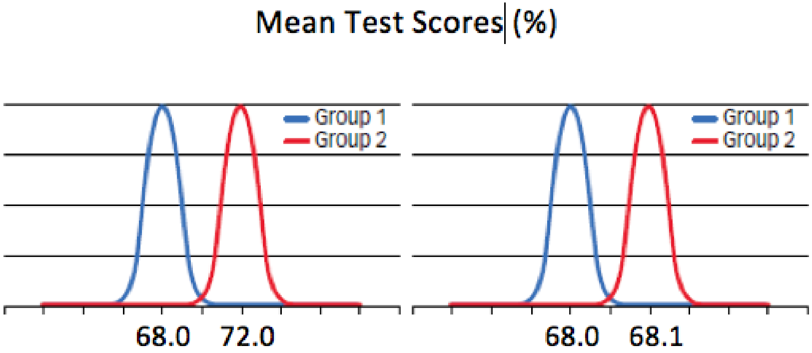
As sample size increases:

- precision of estimates increase
- distribution of statistics become more predictable
- power of a statistical test increases
- estimated width of a confidence interval decreases



Required Elements of the Sample Size Calculation

Matching statistical significance with results that are important



The minimum effect you care about finding (effect size)

Two Kinds of Errors

I and II error.jpg

Want to minimize these two kinds of errors

Required Elements of the Sample Size Calculation

The magnitude of the two types of error:

- The chance of concluding an effect exists when there isn't one (alpha, α)
- The chance of not finding evidence for an effect when there is one (beta, β). Note that 1 - β is the Power, the chance of finding an effect when there is one.

And sometimes the knowledge of the variability in the parameters of interest

On-line Demonstration sample size and power

Russ Lenth on-line java applets

Select the sample size that yields the desired power or confidence interval width

Power and Error Limits

Other resources:

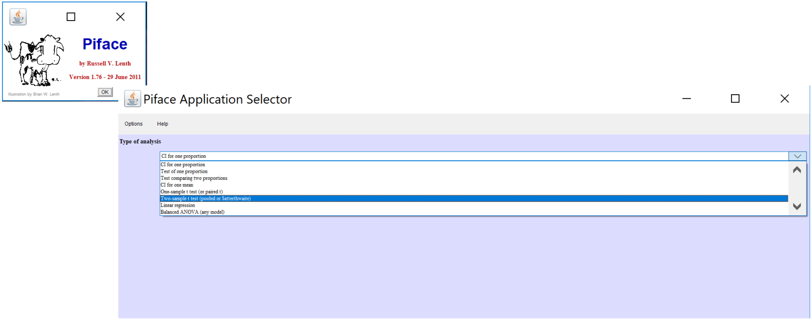
R has built in functions for anova, proportions and t-test plus packages like *pwr*, *longpower* and many others.

Wikipedia -> **Statistical Power**

- 7. Software for Power and Sample Size Calculations

On-line Demonstration sample size and power - cont.

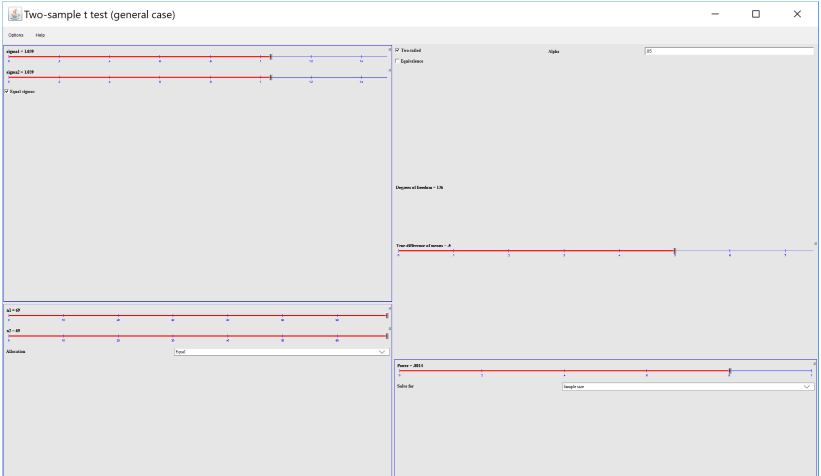
Russ Lenth on-line java applets - two sample t test



On-line Demonstration sample size and power - cont.

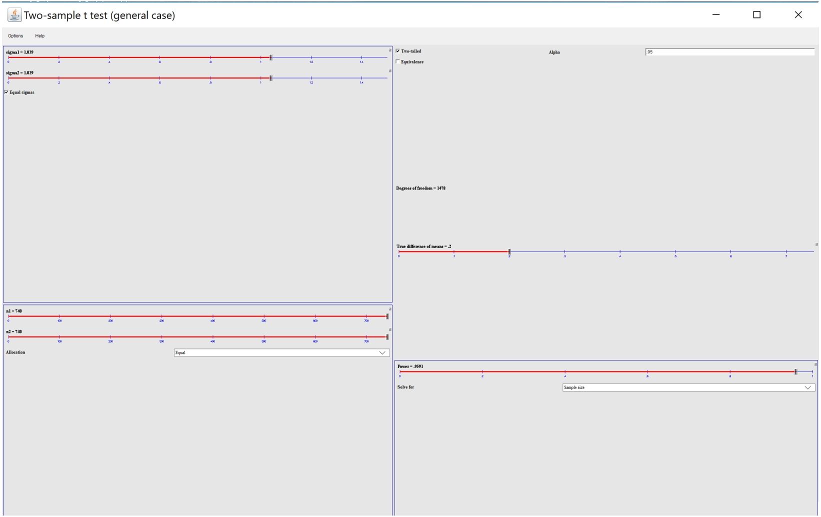
Russ Lenth on-line java applets - two sample t test, play with $sigma_1$, $sigma_2$, effect size, power and see how the sample size changes.

- To detect effect size=0.5 with power of 0.9, sample size needed is $n_1 = n_2 = 69$



On-line Demonstration sample size and power - cont.

- To detect effect size=0.2 with power of 0.95, sample size needed is $n_1 = n_2 = 740$



Statisticians can help

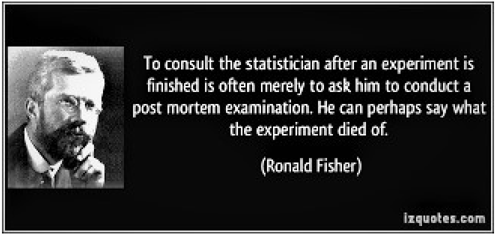
Focus and clarify the objectives

Design an appropriate sampling plan

Provide a randomization scheme

Design an appropriate analysis plan

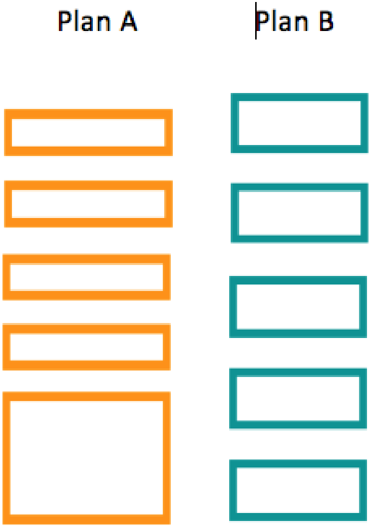
Calculate the sample size or power



Talk to a statistician before you collect your data !!!.

Experimental Design

Balance is better



Power of balanced designs

4 treatments versus control: total 40 experimental units

- Plan A: assign 5 to each treatment and 20 to control
- Plan B: assign 8 to each treatment and 8 to control
- Equally efficient for testing treatment versus control
- B is 60% more efficient when comparing treatments to each other
- B is an overall better design

Covariates

Often collected in addition to the measured outcome of interest

Observational and hard to design into the experiment

May be a confounding or interacting variable

Effect of Exercise (Ex) on Blood Pressure (BP)

Pt	Ex	BP	Age	Weight	BSA	Pulse	Stress
1	Yes	105	47	85.4	1.75	63	33
2	No	115	49	94.2	2.10	70	14
3	No	116	49	95.3	1.98	72	10
4	No	117	50	94.7	2.01	73	99
5	Yes	112	51	89.4	1.89	72	95
6	No	121	48	99.5	2.25	71	10

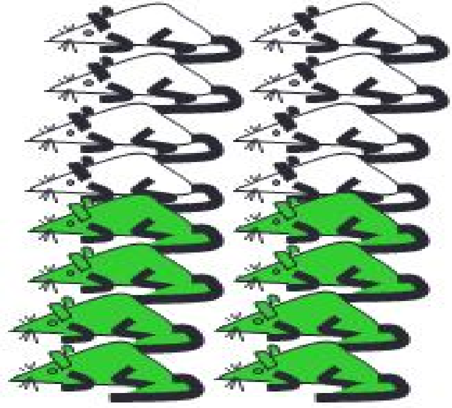
Including highly correlated predictors in the analysis can elevate the risks of false-positives and false-negatives

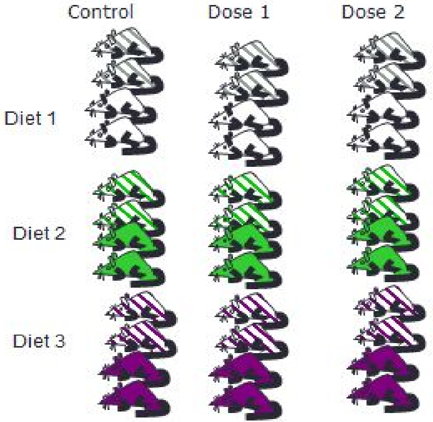
Blocking

New Product
Existing Practice
Existing Practice
New Product
Existing Practice
New Product
New Product
Existing Practice

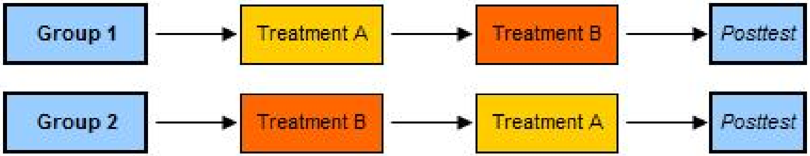
Factorial design

When there is more than one factor





Within Subject designs

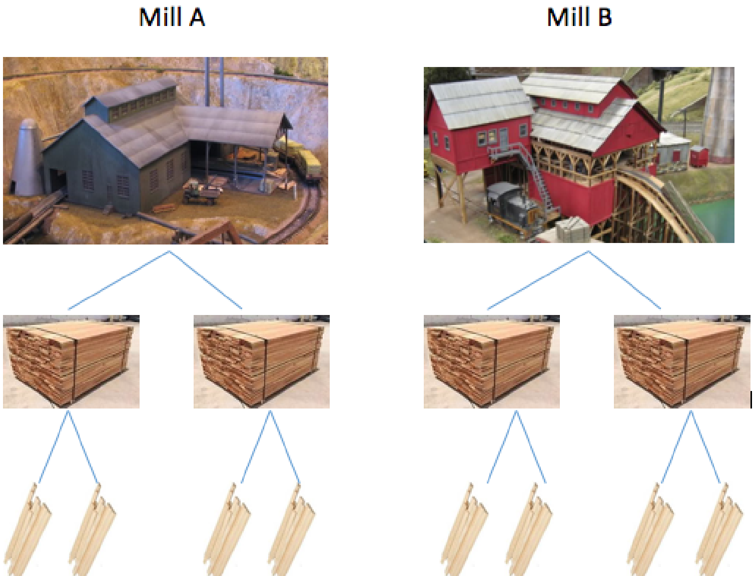


A subject is exposed to more than one treatment

- It eliminates the between subject variability
- Treatment order needs to vary between different subjects
- With many treatments a latin square design can be used for counterbalancing
- Even with counterbalancing, there can be carryover effects
- If carryover effects exist then the study results can be unreliable

Hierarchical design

Clustered samples are not independent



Experimental Design

Balance eliminates confounding and increases power

Correlated predictors can cause a loss of statistical power

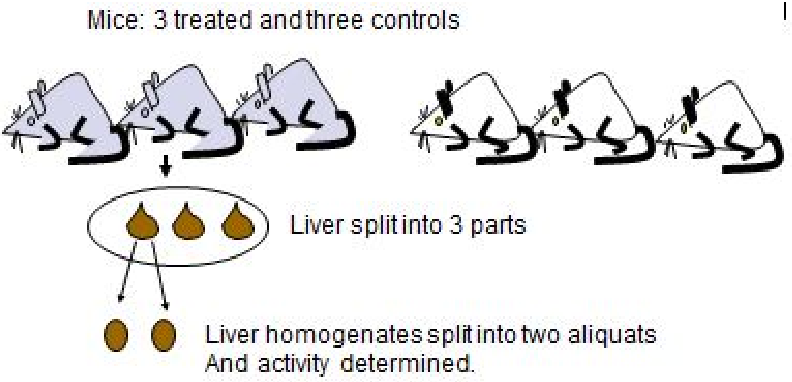
Consider blocking factors to control variation. Make sure groups vary within each block

Make sure all combinations of factors are observed (factorial design) otherwise confounding and evaluating interactions can be a problem

Repeated measurements within a subject has a diminishing return except when evaluating predictors that vary within each subject (multiple treatments)

Be aware of any hierarchical structure in your data. Make sure your analysis controls for any such structure (mixed models)

Experimental Units



What are the experimental units?

True replicates

Have the most influence on the power to test for an effect

Determined by the experimental unit

- usually the level at which the treatment is assigned
- considered to be independent of each other

Pseudo-replicates

- observations within an experimental unit
- can be used but require a more complicated analysis to obtain accurate results

Multiple Comparisons

Often researchers are not interested in just one treatment versus control comparison per experiment

Comparisons proliferate with interest in:

- more than 1 treatment, there are t(t-1)/2 possible comparisons
- effects within subgroups
- more than one distinct outcome

Multiple Comparisons

Example of the effect on the chance of a false positive:

- with three levels (2 treatment, 1 control), 3 possible comparisons
- within two subgroups (adult and children), up to 6 comparisons
- repeat for two outcome measures of interest, up to 12 comparisons
- each comparison is tested separately at $\alpha = 0.05$
- probability of at least one false positive in the 12 tests is 0.46

Family-Wise Error Rate (FWER) is the chance of at least one false positive in the multiple comparisons or tests

Controlling the FWER

When you want to guard against any false positives

Commonly used Bonferroni correction for k tests (α/k) is very conservative

Example:

- For 12 tests, the significance level of 0.05 / 12 = 0.004 is used for each test
- guarantees chance of any false positive is $\alpha = 0.05$

Less conservative to control the False Discovery Rate (FDR) - proportion of false discoveries among all discoveries

- when a certain number of false positives is ok
- less stringent control of Type I error compared to FWER methods

Recommendation: limit the number of questions or select a few questions as primary beforehand

Types of variables

Data can play the role of the response variable (dependent, Y) or the explanatory variable (independent, X)

Data can be numeric (continuous or discrete). This is a real number with a real interpretation

Data can be categorical, which can be ordinal (small, medium, large) or nominal (red, green, blue)

The type of analysis depends on which type of variable is in each role

- The response variable is most important

Common Analyses

Continuous response variable

- t-test, regression, ANOVA, ANCOVA

Response variable is a count

- Poisson or negative binomial regression

Binary response variable

- contingency tables, logistic regression

Categorical responses are more complicated

- ordinal or nominal logistic regression

Questions?

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