## Statistical Methods



**Inferential Statistics:** science of using the **information in your sample** to say (i.e. to "**infer**") something **about the population** of interest

More precisely, to infer or estimate the value of population parameters using sample statistics as estimates

We will come back to this later!

**Descriptive Statistics:** Science of summarizing data, numerically and graphically...

Analysis methods applicable depends on the variable being measured and the research questions which you are trying to answer ... How would you collect the data?



### Key concepts

- Population
  - *A parameter* is a single value summarising some feature of variable of interest in the *population*
  - It is usually unknown...

### • Sample

- *A statistic* is a single value summarising the observed values of the variable from the *sample* collected
- Sample statistics will vary from sample to sample
- Source of uncertainty....



### **Inferential Statistics:**

*Inference* is the process of making decisions about a population based on information in a sample

Science of Inferential Statistics:

to infer or estimate population parameters using sample statistics



Take a representative sample of 5 circles from the population of 60 circles and use the sample mean as an estimate of the true population mean









A consequence of <u>natural variation</u> is that two samples drawn from the same population will usually give different estimates of the population parameters

Referred to as **sampling variation** 

How can we choose a representative sample of size 500 University of Galway students?



*Non-probabilistic sampling* methods are techniques of obtaining a sample that is not chosen at random and may be subject to **sampling bias**.

### Examples of Convenience Sampling

- In the *amount spent on rent weekly* problem sending emails to students in the hope of a response may give this result
- Could sample by standing at entrance to college bar or on concourse another example of convenience sampling

### An issue with Convenience Sampling

- Leave it to experimental unit to choose to complete a survey or opinion poll
  - A response may be more likely to be received because a responder has a particularly strong opinion
  - If sample consists of mainly such strong opinions, then sample may not be representative of population



Simple Random Sampling

"Subset of *n* units chosen from population of *N* units, chosen randomly so that every unit has same chance of selection"

Or equivalently, every randomly subset of same size has same change of being observed

## Sound easy...

## Just "table and label"!



### https://istats.shinyapps.io/RandomNumbers/



## Simple random sample

### **Difficulties:**

- Obtaining a sampling frame (list of all experimental units)
- Possibly time consuming / expensive
- Minority groups, by chance, may not be represented in sample

e.g. population of N = 17,520 University of Galway students,

400 of which are mature students

how many in sample of n = 500?



## Stratified Random Sampling

(i) Split entire population into homogeneous groups, called strata(ii) Take a SRS from each stratum

### **Proportional Allocation**



## Stratified Compared to SRS

- Ensure representation from minority groups
- Estimates of the population parameters per strata may be of interest
- Possibly reduction in cost per observation in the survey
- Increased accuracy as reduced sampling error (less variation within a stratum)

# Stratified Compared to SRS

- Can you correctly allocate each individual to one and only one stratum?
- Should every group receive equal weight?
- What if some strata are more varied than others?
- Take into account mean, variance and cost to get "optimal allocation"

# What if a sampling frame (or strata criteria) is unavailable?

# Non Probability Sampling Probability Sampling SRS Sratified

## **Cluster Sampling**

Instead of randomly choosing individuals,

a SRS of collections or groups of individuals is taken

# **Cluster Sampling**

Population is broken up into regions or groups, usually a natural partition, called a *cluster* 

e.g., geographical areas, or a class!

- Clusters are assumed representative of entire population (internally heterogenous, between are homogeneous)
- Small number of clusters are selected at random
- **Every individual** within a cluster are observed

### Note:

in stratified sampling all strata are sampled while in cluster sampling only some clusters are sampled

This is a crucial point!

# Cluster over Stratified

- Sampling frame not necessarily needed
- May be more practical and / or economical than SRS or stratified sampling
- Will be biased if entire cluster not sampled
- Careful if homogeneity within cluster and heterogeneity between clusters as this can increase sample error

## Summary

- Try to estimate population parameters with sample statistics
- Want sample to be as representative of the population as possible
- Probability based sampling schemes are best in terms of minimising chance of bias

# Typical Exam Questions

1. Which of the following statements is true regarding a population:

- a) it must refer to people;
- b) it is a collection of individuals or objects;
- c) neither of the above.

# Typical Exam Questions

- 2. A sampling frame is:
  - a) the list of units from which the sample is chosen;
  - b) a table of random numbers;
  - c) a non-probabilistic sampling method.

# Typical Exam Questions

- 3. Sampling that divides the population in subgroups and chooses a proportionate number from each subgroup at random is called:
  - a) cluster sampling;
  - b) quota sampling;
  - c) stratified sampling.

# Collecting the Data (Sampling)

- Observational Study
- Designed Experiment

## Observational studies & experiments

- Observational study:
  - data collected only by observing what occurs (e.g. surveys, historical records)
- When researchers want to investigate causal relationships best to conduct an experiment
  - Usually there will be both an explanatory and a response variable
- Be wary of confounding variables.

# Designed (comparative) Study

- An experiment allows us to prove a cause-and-effect relationship
- Experimenter must identify:
  - at least one explanatory variable, called a factor, to manipulate; and
  - at least one response variable to measure
- They must control any other nuisance factors that could influence the response, e.g. weather, day of week, ...

## Designed (comparative) Study

- An experiment allows us to prove a cause-and-effect relationship
- An experiment will:
  - Define treatment factor(s)
  - Randomly assigns subjects to treatment levels
  - *Compares* responses of the subject groups across treatment levels
- Key difference between an observational study and an experiment is that in an experiment we apply a treatment to the subjects in a controlled way

### Comparative Studies (Independent Samples)



- Randomisation
- Control Group
- Baseline (Pre) measurement
- Blinding
- Replication, not pseudo-replicates

### Designed Study (cont.)

- When humans are involved, the term "experimental units" is commonly replace with subjects or participants
- A treatment is a combination of specific levels from all the factors that an experimental unit receives
- A baseline measurement is the initial measurements of the response at the beginning of the experiement
- Changes from the baseline due to varying the treatment level are of usually interest
- A control group receive a standard treatment (usually a <u>placebo</u>, or no treatment at all) called a <u>control treatment</u>, often the response is not expected to change from baseline for the control group

# Principles of Study Design

- Controlling (not just control group)
- Randomisation
- Replication
- Blocking (stratifying)
- Blinding

#### CHAPTER 1. INTRODUCTION TO DATA

#### 1.5.1 Principles of experimental design

#### Randomized experiments are generally built on four principles.

- Controlling. Researchers assign treatments to cases, and they do their best to control any other differences in the groups. For example, when patients take a drug in pill form, some patients take the pill with only a sip of water while others may have it with an entire glass of water. To control for the effect of water consumption, a doctor may ask all patients to drink a 12 ounce glass of water with the pill.
- Randomization. Researchers randomize patients into treatment groups to account for variables that cannot be controlled. For example, some patients may be more susceptible to a disease than others due to their dietary habits. Randomizing patients into the treatment or control group helps even out such differences, and it also prevents accidental bias from entering the study.
- Replication. The more cases researchers observe, the more accurately they can estimate the effect of the explanatory variable on the response. In a single study, we replicate by collecting a sufficiently large sample. Additionally, a group of scientists may replicate an entire study to verify an earlier finding.
- Blocking. Researchers sometimes know or suspect that variables, other than the treatment, influence the response. Under these circumstances, they may first group individuals based on this variable into blocks and then randomize cases within each block to the treatment groups. This strategy is often referred to as blocking. For instance, if we are looking at the effect of a drug on heart attacks, we might first split patients in the study into low-risk and high-risk blocks, then randomly assign half the patients from each block to the control group and the other half to the treatment group, as shown in Figure 1.16. This strategy resures each treatment group has an equal number of low-risk and high-risk patients.

## Principles of Experimental Design

### • Controlling:

• Need to control all nuisance factors that may influence response or sources of variation, other than the treatments of interest by making conditions as similar as possible for all treatment groups

#### • Randomisation:

- Attempts to "equalise" the effects of unknown or uncontrollable sources of variation
  - <u>It does not eliminate the effects of these sources</u>, but it tries to minimise their impact
- Without randomisation, you do not have a valid experiment and will not be able to use the powerful methods of statistics to draw conclusions from your study

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# Principles of Experimental Design

### • Replication:

- Repeat the experiment, applying the treatments to a number of subjects
- Blocking (stratifying):
  - Sometimes attributes of the experimental units, that we are not studying, and that we can't control may affect the outcomes of an experiment
  - Solution: group similar individuals together and then randomise within each of these blocks, to remove much of variability between the blocks
  - Note: blocking is an important compromise between randomisation and control but is not *required* in an experimental design

# Blocking

- When experimental units within a group are similar, it's often a good idea to gather them together into blocks
  - Blocking isolates the variability due to the differences between the blocks so that we can see the differences due to the treatments more clearly
  - Basically it removes a source of noise so signal stronger
- When randomization occurs only within the blocks, we call the design a randomized block design
- Blocking is same idea for experiments as stratifying is for sampling

# Principles of Experimental Design

### • Blinding:

- Refers to the concealment of treatment allocation, from one or more individuals involved in a study
  - Although randomisation minimizes differences between treatment groups at the start of the study, it does nothing to prevent differential treatment of the groups during trial, or the differential assessment of outcomes, which may result in biased estimates of treatment effects
- Best practice to minimise the likelihood of differential treatment or assessments of outcomes is to blind as many individuals as possible in a trial (e.g. participants, experimenters, statisticians)

# Blinding

- There are two main classes of individuals who can affect the outcome:
  - those who could influence the treatment response (usually the subjects, treatment administrators or technicians)
  - those who evaluate the results (statisticians, researchers, physicians, etc.)
- When all individuals in *either one* of these classes are blinded, an experiment is said to be single-blind (usually the first class)
- When everyone in *both* classes is blinded, the experiment is called double-blind

# Placebos

- Often simply applying *any* treatment can induce an improvement
- To separate out the effects of the treatment of interest, we can use a control treatment that mimics the treatment itself
- A "fake" treatment like the treatment being tested is called a placebo (e.g. saline solution or inert pill)
- Placebos are the best way to blind subjects from knowing whether they are receiving the treatment or not

# Placebo Effect

- A placebo effect occurs when taking the sham treatment results in a change in the response variable
- Just being involved in an experiment can change behavior or feelings
- Highlights both the importance of effective blinding and the importance of comparing treatments with a control
- Placebo controls are so effective they should be considered an essential tool for blinding whenever possible

# Best Practice for Experiments

- Usually:
  - randomised
  - comparative
  - double-blind
  - have control group (either placebo or a standard treatment)

# **Observational Studies**

In an observational study we may compare units that **happened** to receive different treatments

### Example: Smoking & Lung Cancer

- Simply comparing across groups that smoked or not
- No control of treatment allocation
- Are those prone to smoking also naturally prone to lung cancer?
- Could identifying "possible" causes, but cannot establish causation

Only properly designed and executed experiments can reliably demonstrate cause-and-effect

# What Can Go Wrong?

- Don't give up if you can't run an experiment
  - If we can't perform an experiment, an observational study may be good option
- Beware of confounding; some other unmeasured variables that has an effect on the response variable intertwined in the experiment (e.g. severity of disease at baseline)
- Use randomisation whenever possible to minimise risk of confounding
- Always report any possible unavoidable confounding

# What Can Go Wrong?

- Bad things can happen even to good experiments
  - Protect yourself by recording additional information
  - Account for nuisance factors in your modelling, even if you randomised
- Don't spend your entire budget on the first run
  - Run a small pilot experiment first
  - You may learn some things that will help you make the full-scale experiment better

## Proposed Design



# Pilot Study







### Summary

- Observational studies are tricky to analyse
- Experimental studies are the key to establishing cause and effect
- Both observational and experimental studies need randomisation to collect unbiased data. But they do so in different ways and for different purposes:
  - Observational studies attempt to randomly select participants from the population.
  - Experiments are usually done by randomly assigning the treatments to the experimental units (e.g. patients) to reduce bias.
- No Control no Experiment
- Carry out a Pilot study
- Consult your favorite Statistician (bring gifts)