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Statistics for HDR Students

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100 YEARS AHEAD

Statistical Analysis involves pooling information:

Aggregating information over individual patients/participants who may vary in

- measurable ways: age, sex, severity, other concurrent illnesses, specific symptoms and signs, health behaviour, social and cultural background, genotypes…..

- non-measureable (unknowable) ways:

This variability contributes to (among other things) **uncertainty** in the 'correct' conclusion from a particular study

‘Non-statistical’ Uncertainty

Judgmental uncertainty

- A large trial shows aspirin prevents heart attacks in men. Will it be the same for women?

- A study shows people with pancreatic cancer report higher levels of smoking than those who don’t have pancreatic cancer. Does smoking cause pancreatic cancer?

Resolving these kinds of uncertainties is partly a matter for argument

Might take into account strength of evidence presented, biological plausibility, and other matters

Uncertainty as 'natural' variation

Statistical uncertainty – specific examples

- Based on a sample of 100 patients, the proportion of patients with a post-op complication after knee replacement is 25%. How precise is this estimate? Is it good enough? For what?

- A study of 60 schools across Australia shows children living in areas of poor air quality have 20% increased risk of asthma. Is this likely to be due to chance?

Resolving uncertainties is largely a matter of using a mathematical model, which models probability distribution relevant to the observed study and allows assessment of the ***role of chance*** in producing the observed findings

A General Framework for Research – and where statistical inputs are needed

The Steps in Research

- What is the research question?
- Can/should the question be deconstructed to smaller, answerable components?
- What design will answer this (these) questions (efficiently)?
- What (how much) data do you need to collect?
- How will you analyse it?
- How will you interpret and present the results?
- What are the limitations? Next steps?

A common situation in practice-based research

- Have access to a database of information collected on a series of patients/presentations/persons in community
- What questions can be answered by the data?
- What kind of design will this be?
- Is there enough data?
- Are there questions about data validity, reliability? Are the data complete, sufficiently well-recorded? Will it need to be re-organised?

Study design

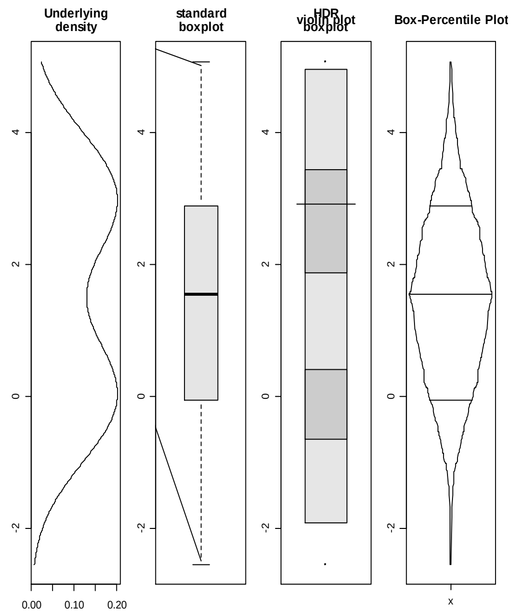
Observational or experimental?

Observational

- Official statistics (secondary data)
- Longitudinal
- Case-control study
- Survey (random)

Experimental

- Randomised (blinded) controlled trial
- Comparative (experimental) study



Looking ahead

What will my results look like?

Research question

Example: Is there a real difference in neck movement (flexion - degrees) between female and male subjects in a study?

	Female	Male
Sample size	100	57
Mean	**40.36**	**43.72**
SD	11.65	10.64

Hypothesis testing

Null hypothesis: no real difference between groups

Alternative hypothesis: real difference (i.e. difference is not zero)

Test statistic:

$$z = \frac{\text{difference in means}}{\text{SE of difference}}$$

$$= \frac{-3.36}{1.875} = -1.79$$

p-value = probability of getting a value as large as this (in either direction) if null hypothesis is correct.

p-values and hypothesis testing

Null hypothesis is

'innocent until proven guilty'.

Require proof beyond 'reasonable doubt'

Conclude there is a real effect only when the observed outcomes are highly unlikely <u>if the null hypothesis (no effect) were true</u>

'Highly unlikely' means a small P-value, usually less than 0.05

Warning!

Differences may be *statistically significant* even if the effect size is small (ie. not of *practical/clinical significance*) - due to large sample sizes

Differences may be *clinically significant* but not *statistically significant* if samples are small or measurements are imprecise (standard deviations are large)

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Sample Size, Precision, Power

100
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Ethical Principle:

Sample size must be

- Sufficiently large to have reasonable chance of answering the research question convincingly
- Have minimal cost in terms of $$, cost of delayed decision, participant time/discomfort, other resources

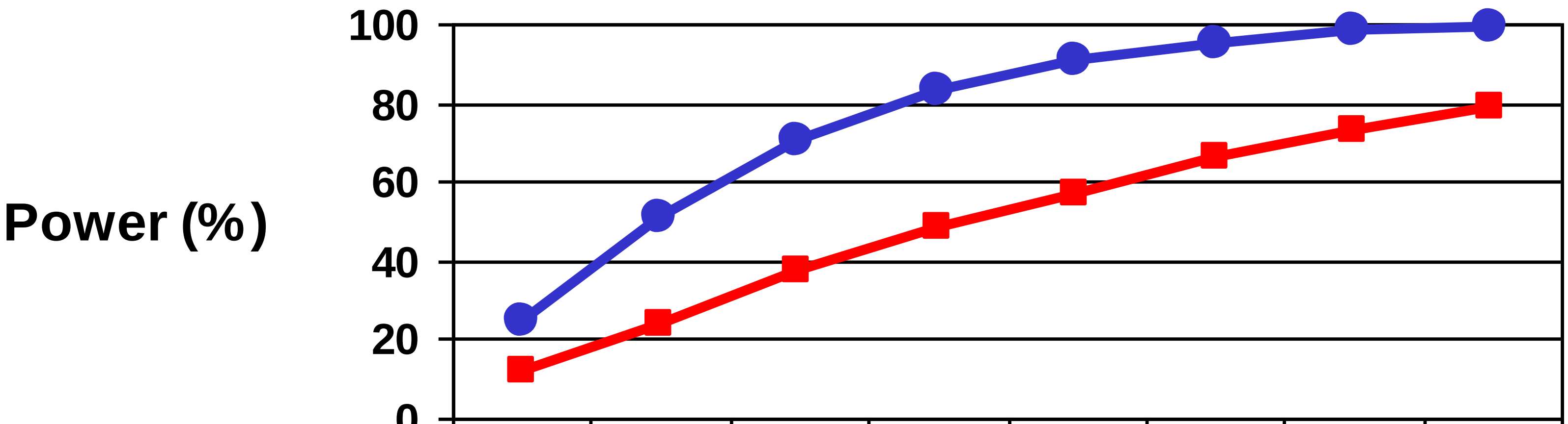
Study is unethical if it is

- Smaller than necessary to answer the question, because it is likely to cause confusion and waste resources
- Larger than necessary to answer the question, because it wastes resources

If a study of the required size cannot be carried out with the available resources then it may have to be abandoned

Power curves

Power (%) (chance of getting a significant result from the study) for various sample sizes in each group when comparing 40% vs 55%, and 45% vs 55%



	50	100	150	200	250	300	350	400
40% vs 55%	25	51	70	83	91	95	98	99
45% vs 55%	12	24	37	48	57	66	73	79

Sample Size N

Types of comparative analyses

Next 4 slides: FYI

Review of statistical techniques

Problem	Analysis
Compare two proportions, when samples are independent	Chi-squared test (with 1 degree of freedom) Fisher's Exact test (small samples)
Examine relationship between two categorical variables - independent observations of the two cross tabulated categorical variables	Chi-squared test for $r \times c$ table, r = number of rows, c = number of columns
Compare two proportions, when samples are paired, as in before-after data	McNemar's test

Review of statistical techniques

Problem	Analysis
Examine strength of linear relationship between two continuous variables (Normally distributed)	Pearson Correlation coefficient
Variables not Normally distributed Examine strength of linear relationship between two continuous	Kendall rank correlation coefficient, or Spearman's rank correlation coefficient
Examine how much one continuous variable (Normally distributed) changes linearly with changes in another continuous variable	Regression analysis

Review of statistical techniques

Problem	Analysis
Compare two means, of a Normally distributed variable, from independent samples	Two-sample t-test
Compare two means, based on non-Normally distributed variables from independent samples	Transform variables, or use Wilcoxon-Mann-Whitney test
Compare ≥ 2 means, based on Normally distributed variable, and independent samples	Analysis of Variance
Compare ≥ 2 means, based on non-Normally distributed variables and independent samples	Transform variables, or use Kruskal-Wallis Analysis of Variance

Review of statistical techniques

Problem	Analysis
Compare two means, based on Normally distributed variable, when samples are paired, as in before-after data	One-sample t-test
Comparing two means, based on non-Normally distributed variable, when samples are paired, as in before-after data	Transform variables, or use Wilcoxon Signed Rank test
Compare ≥ 2 means, based on Normally distributed variable, and repeated measures	Repeated measures Analysis of Variance

Common mistakes

- Objectives unclear or not prioritised – connection between research question, design and analysis
- Insufficient attention to/resources for planning data management
- Not understanding the connection between data management and analysis (beware spreadsheets!)
- Lack of consideration given to sample size – ask the right question!
- Presentation: Inadequate description of sample and sampling frame
- Overemphasis/misinterpretation of P-values
- Not allowing for resources/time for analysis

Statistical support provided by the School of Public Health

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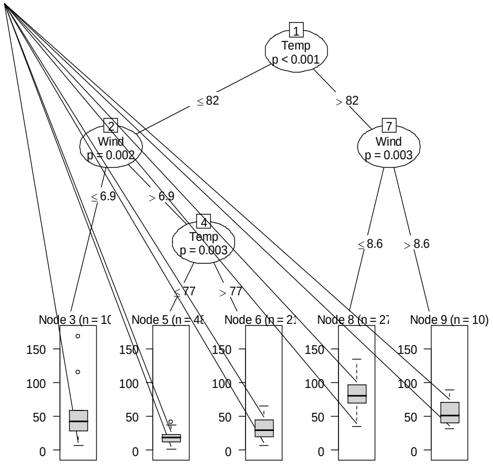
We can provide advice on

- Study and survey design
- Random samples or allocation
- Setting up data for analysis and presentation
- Statistical software SAS, R, stata, SPSS, ...
- Data analysis and presentation of results
- A conduit to more specialised advice
- Courses and resources



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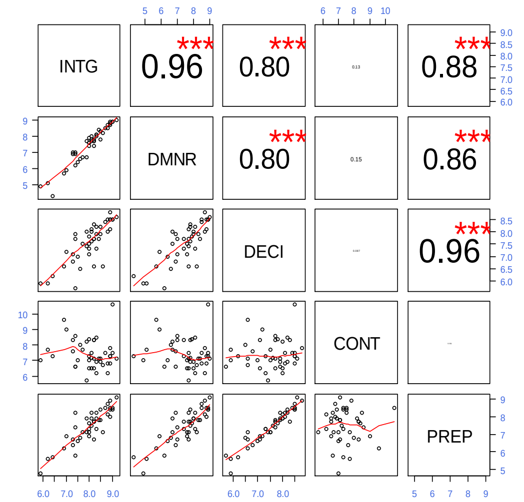
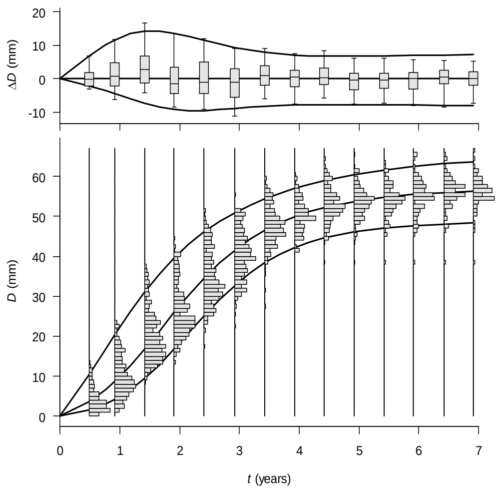
Final points

- A good study design is critical
  - » subject / sample selection
  - » appropriate measurements
  - » appropriate treatments
  - » power and significance
  - » sample size
  - » make goals achievable
- MPH courses – need to enrol
  - » PUBH7630: Introduction to Biostatistics
  - » PUBH7631: Practical Regression Analyses
  - » PUBH7632: Advanced Epidemiology and Biostatistics



How to contact us



Fill in the appropriate “Request for Statistical Support” form available from Alison Manly
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