Biostats and Biases

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Disclosures

• No relevant disclosures
What is “statistics”?  

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

**Google:** the practice or science of collecting and analyzing numerical data in large quantities, especially for the purpose of inferring proportions in a whole from those in a representative sample.

*synonyms:* data, facts and figures, numbers, information, details
Why Do Surgeons Need to Know About Statistics?

• Understand literature for clinical care
• Develop appropriate research questions and approaches
• Communicate with statisticians and other scientists
Goals of this talk

• Will help with some vocabulary
• Demonstrate some skills for communicating intelligently with biostatisticians, analysts, and others
• Encourage you to learn more
Disclaimers

• I am not a biostatistician
• Will not teach you to do statistics today
• There will be almost no numbers or complex equations presented
• No test at the end
From observation to understanding

- Observations create data
  - Experimental
  - Ecological
- Data can be truth but not necessarily so
  - Role of chance
  - Role of bias
  - Reliability of observations
Define your data

- Continuous
- Categorical
  - Binary
  - Ordinal
  - Nominal
- Dependent vs Independent Variables (e.g. Outcome vs Predictor)
Definitions

- Null hypothesis: there is no difference in outcome between the two exposures
- Type I error = false rejection of the null hypothesis ($\alpha$)
- Type II error = false acceptance of the null hypothesis ($\beta$)
- Power = $1 - \beta$
When should you engage with a statistician?

• Early and often!
• When writing protocol or proposal
• Design analysis ahead of data collection
• Ensure that methods are able to answer the question that you have
  – Power
  – Types of data
How can my study be powerful?

• Power = 1 - β
  – Set at 80 (pilot) – 95% (final, conclusive study)
  – 90% is accepted power for NIH funding
• Goal is to minimize both Type I & II error to maximize the chance that observation is true
Power/Sample Size Calculations

- You need 3 of the following 4:
  - Sample size (N)
  - Power (1 – β)
  - Pre-specified surety (α)
  - Effect size

- What is effect size?
  - Difference between 2 means, times, or proportions
  - Based on pilot data, best guess, “minimum clinical significance”
Power/Sample Size Calculations

• *A priori* analysis
  – Determine the sample size necessary to detect a clinically important effect with pre-specified surety
  – Determine the chance that with a given sample size, a clinically significant effect can be detected
  – Determine the magnitude of the effect size that can be detected with a pre-specified surety and sample size

• *Post hoc* analysis
  – Once the study is done, determine the probability that the conclusion is correct

*Orav, 2002*
What is significant?

• Statistical significance
  – Data are extreme enough to reject null hypothesis at present level of significance ($\alpha$), typically 0.05

• Clinical significance
  – Is the level of difference identified meaningful for clinical use
  – Varies by instance
  – Take into account imprecision and generalizability
  – Different for medicine and public health
0.05 or bust!

• A p-value is the conditional probability of obtaining the results in hand if the null hypothesis is true
• In plain English, how likely are you to get these results if there is truly no difference?
• Significance (α) is set *a priori* and represents the likelihood of falsely rejecting the null hypothesis
Some Basics for Analysis

• Is the outcome categorical or continuous?
• Is the predictor categorical or continuous?
• Is the data paired?
  – Sequential measurements made on the same subject
    • HR before and after medication X is given
  – Need to account for the fact that these are likely to be more highly correlated
  – If do not do paired analysis, you can introduce bias into significance of results
Some Basics for Analysis

• Is the data normal?
  – If yes, then
    • Parametric tests = Assumes that the data are drawn from a normal distribution
  – If no, then
    • Non-parametric = No such assumption
      – Based on the rank order
    • Transform data to make it normal and use a parametric test
      – Logarithm, square root, reciprocal
Fig 1 Normal

Fig 2 Skewed
Univariate Analysis

• Single outcome with single predictor
• Many different tests, depending upon:
  – Number of groups
  – Relationship of groups
  – Nature of outcome and predictor (discrete, normal, non-normal)
  – Examples: Chi-Square, Fisher exact, t-test, Wilcoxon Rank Sum, ANOVA, Spearman, Pearson, Kruskal-Wallis, McNemar
<table>
<thead>
<tr>
<th>Discrete Outcome</th>
<th>1 Sample (Paired)</th>
<th>2 Samples</th>
<th>3 or more Samples</th>
<th>Continuous Predictor</th>
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<tbody>
<tr>
<td>McNemar’s Test</td>
<td>Fisher/Chi-square</td>
<td>Fisher/Chi-square</td>
<td>T-test/ANOVA</td>
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<td>Normal Outcome</td>
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<td>ANOVA</td>
<td>Pearson Spearman</td>
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<td>Non-Normal Outcome</td>
<td>Wilcoxon Sign Rank</td>
<td>Wilcoxon Rank Sum</td>
<td>Kruskal-Wallis</td>
<td>Spearman</td>
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Multivariable modeling

- Build a model that relates the predictors to the outcome through a mathematical equation
  - Adjust for confounders
  - Build a prediction model
- Types of multivariable modeling
  - Linear regression
  - Logistic regression
  - Cox regression
Validity

• Internal validity – do the results represent a real relationship?
• External validity – can the study be applied to "real life"?
Biases

• Selection bias
  – Selection affects external validity
  – Selection bias affects internal validity

• Information bias
  – Misclassification bias
    • Differential misclassification changes findings
    • Random misclassification reduces power
  – Recall bias
  – Reporting bias
  – Surveillance bias
Confounding

• An exposure is a confounder if:
  1. It is a risk factor for disease and
  2. It is associated with A but not a result of A

• Example
  – Coffee drinkers have a higher rate of pancreatic cancer
  – However, this is because cigarette smoking, a known risk factor for pancreatic cancer, is more prevalent amongst heavy coffee drinkers
Approaches to confounding

- Matching
- Stratification
- Adjustment
- Capture of additional data
Statistics are a lens

• Analysis isn’t something that happens after data collection
• Early input from statistical colleagues important
• Understanding biases and confounding essential to interpreting results
• Many opportunities to expand understanding
Thank You

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Use this slide if you need to transition between topics.
### Color Palette

<table>
<thead>
<tr>
<th>Color</th>
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