Outline

1. Descriptive Statistics
2. Limited Role for Hypothesis Testing and $P$-values
3. Bias and Precision
4. How to Present Results
5. Respecting Continuous Variables
Descriptive Statistics

- Best not to assume shape of distributions
- Let the data speak for themselves
- Three-number summary: 25th, 50th (median), 75th percentiles
- Describes central tendency, spread, symmetry
Hypothesis Testing

- Existence of ESP is a hypothesis
- Assessing effects of drugs, procedures, devices involves estimation
- Many studies powered to detect huge effect
- If effect is not huge, no information from study
- Provide evidence against a *null* hypothesis

- Probability of a statistic as impressive as yours if $H_0$ true

- Not a probability of an effect or difference (same problem with sensitivity)

- **No** conclusion possible from large $P$-values

- Cannot conclude clinical relevance from small $P$
• Best addressed with study design
  – randomization
  – minimize work-up or referral bias

• Sometimes handled by careful regression analysis
  – adjust for patient selection
  – adjust for confounding risk factors
• Erroneous estimates caused by bias and imprecision

• Precision = margin of error

• Standard error or $\frac{1}{2}$ width of confidence interval if estimate is unbiased

• Margin of error $\downarrow$ as $n \uparrow$
How Not to Present Results

- $P = 0.02$ — let’s put this into clinical practice
- $P = 0.4$ — this drug does not kill people
- $P = 0.2$ but there is a trend in favor of our blockbuster drug
- The observed difference was 6mmHg and we rejected $H_0$ so the true effect is 6mmHg.
• The proportion of patients having adverse events was 0.01 and 0.03; the study wasn’t powered to detect adverse event differences so we present no statistical analysis.

• The reduction in blood pressure was 6mmHg with 0.95 C.L. of [1mmHg, 11mmHg]; the drug is just as likely to only reduce blood pressure by 1mmHg as it is by 6mmHg.
How to Present Results

- Estimates should be accompanied by confidence limits.
- Confidence limits can be computed without regard to sample size or power.
- A computed value from a sample is only an estimate of the population value.
- Best to think of an estimate from a study as a fuzz, not a point.
Bayesian Presentation

- Posterior probability density (like histogram) for likelihood of effects equaling certain values
- Solves “optical illusion” problem of flat confidence bars
- Is the most intuitive way to communicate evidence
- Can compute probability of a \textit{clinically significant} difference
Bayesian Current Probability Distribution

Evidence for Effect

Effect
Example: Comparing Two Proportions

- Provide the two proportions
- Confidence limits for difference
- Confidence limits for relative difference (odds ratio)
- Bayesian posterior probabilities of these two
Respecting Continuous Variables

- Keep all continuous variables continuous
- Maximizes power and precision
- Cut-points are arbitrary
- Diagnosis: use extent of disease instead of presence
- Prognosis: days until clinical endpoint
- Test output: use actual measurements or degree of positivity
Joint Effect of Age and Cholesterol on Risk of CAD