# Stats Overview for Clinical Researchers

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# 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

# $P$-Values

- 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 $P$

# Bias

- 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

# Precision

- 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.

## How Not to Present Results, cont.

- 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

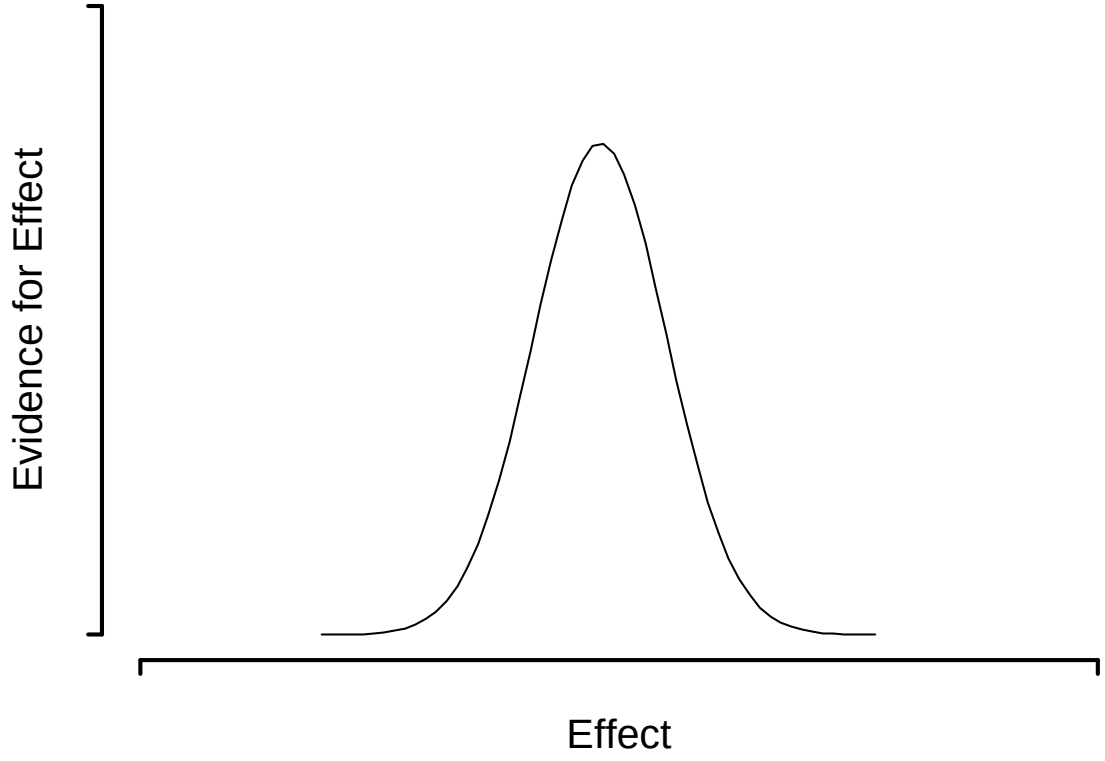


www.phinxphotography.com/paintbrush_dandelion.htm

## 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 *clinically significant* difference

# Bayesian Current Probability Distribution



# 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