Clinical statistics for non-statisticians: Extra topics

Steve Simon

Your comments, 1 of 15

- When do you use randomized versus non-randomized studies?
  - Observational studies
  - Experimental studies
    - Randomized studies
    - Quasi-experimental studies
  - Randomization is overrated
- Definitely will cover

Your comments, 2 of 15

- Different types of clinical trials
  - Crossover trials
  - Adaptive trials
  - Real world evidence
- Might cover

Your comments, 3 of 15

- Superiority vs non-inferiority
  - New drug is
    - cheaper,
    - more convenient,
    - fewer side effects
  - Willing to tolerate a small deficit in efficacy
- Might cover

Your comments, 4 of 15

- Balanced vs unbalanced study design, randomization ratio
  - All others things being equal, balanced is better
  - What to do when controls are cheaper
- Might cover

Your comments, 5 of 15

- Number of matching/stratification factors
  - Don’t go overboard!
  - Only the “drop dead” important factors
- Might cover

Your comments, 6 of 15

- Frequency of assessment
  - Cost versus information trade-off
- Probably won’t cover

Your comments, 7 of 15

- Disproportionate patient drop-out
  - Often a fatal problem
  - Prevention!
  - Get partial information
  - Intention to treat analysis
  - Imputation

Your comments, 8 of 15

- Dose escalation and dose optimization
  - 3+3 designs
  - Bayesian designs
- Probably won’t cover

Your comments 9 of 15

- Mis-stratification error rate
  - Randomization relies on large numbers
  - Physician subversion?
- Probably won’t cover

Your comments, 10 of 15

- How do you handle missing data?
  - No news is good news
  - No news is bad news
  - No news is average news
  - No news is yesterday's news
  - Single imputation
  - Multiple imputation
- Might cover

Your comments, 11 of 15

- Explaining complex statistical concepts
  - Avoid condescension
  - Size up your audience
  - Cite relevant, accessible examples
  - Focus on nouns, not adjectives
- Might cover

Your comments, 12 of 15

- Different ways to visualize data
  - Uses and abuses of color
  - The error of error bars
  - Proximity principle
- Definitely will cover

Your comments, 13 of 15

- Time to event models, survival
  - Censored values are not missing values
  - Kaplan-Meier curve
  - Proportional hazards models
- Definitely will cover

Your comments, 14 of 15

- Number needed to treat, number needed to harm
  - Absolute versus relative risk
  - “Good” values for NNT
  - NNT to NNH ratio
- Might cover

Your comments, 15 of 15

- Bayesian analysis, historical benchmarks
  - Prior distribution
  - Likelihood
  - Posterior distribution
  - Controversies
- Definitely will cover

Give me your feedback

1. To randomize or not to randomize
2. Types of clinical trials
3. Non-inferiority trials
4. Unbalanced trials
5. Number of strata
6. Frequency of assessment
7. Disproportionate drop-out
8. Dose escalation
9. Mis-stratification
10. Missing data
11. Explaining complex concepts
12. Visualization
13. Survival models
14. NNT, NNH
15. Bayesian analysis