Design and interpretation of clinical trials

Week 6 randomized clinical trials

Johns Hopkins @ Coursera

Frameworks for evaluating evidence

Unsystematic clinical observation → physiologic studies → single observational study addressing patient-important outcomes → systematic review of observational studies addressing patient –important outcomes → single randomized trial → systematic review of RCT (randomized clinical trials)

Key strengths of RCT

- Randomization
  - Unbiased assignment of treatment
  - Comparable groups on known and unknown factors
  - Application of statistical methods based on random sampling
- Standardization
  - Treatments (experimental and control)
  - Outcome assessment
- Assume everything else is equal

Quiz

1. A major strength of randomized clinical trials is that they tend to produce comparable treatment groups based on known and unknown baseline factors.

   ○ False

   ◉ True

2. When evaluating the strength of evidence for a specific treatment on a health outcome, the evidence from unsystematic clinical reports typically carries more weight than the evidence from observational studies.

- (●) False
- ( ) True

2. When evaluating the strength of evidence for a specific treatment on a health outcome, the evidence from randomized clinical trials typically carries more weight than the evidence from observational studies.

- ( ) False
- (●) True

3. Observational studies and randomized clinical trials of the same treatment and outcome can sometimes result in conflicting findings.

- (●) True
- ( ) False

4. Comparing results from two trials with different lengths of follow up can result in inconsistent conclusions about the treatment's efficacy between the two studies.

- (●) True
- ( ) False

5. Confounding should be ignored when interpreting the results of a study.

- (●) False
- ( ) True

6. Standardizing follow-up procedures for study participants helps improve precision in randomized clinical trials.

- (●) True
- ( ) False

7. Compared to randomized clinical trials, a strength of observational studies is that the participants may be more diverse and therefore the results may be more generalizable to other patient populations and health care settings.

- (●) True
- ( ) False

8. Continued surveillance of treatments using observational studies are useful for identifying safety signals and potential adverse events that were not detected in earlier clinical trials.

- (●) True
- ( ) False

9. Reporting bias is more likely to be introduced in randomized clinical trials than in observational studies.

- (●) False
- ( ) True

10. In situations where it is not ethical to conduct a randomized clinical trial, observational studies can provide valuable information.

- (●) False
- ( ) True

Correct answer: true

1. A major strength of randomized clinical trials is that the design features always control for all types of bias within the study design.

- (●) False
- ( ) True

5. Confounding can lead to misleading interpretations of the results of a study.

- ( ) False
- (●) True

6. Observational studies tend to produce more precise estimates of the true treatment effect compared to randomized clinical trials.

- ○ True
- ◉ False

8. From a practical perspective (e.g. time, funding), randomized clinical trials are often more appropriate for evaluating the long-term effects of a particular treatment compared to observational studies.

- ○ True
- ◉ False

9. Selection bias is more likely to be introduced in observational studies than in randomized clinical trials.

- ○ False
- ◉ True

10. Observational studies are of little value to investigators and should not be pursued because there is a high likelihood that bias can be introduced.

- ○ True
- ◉ False

4. Comparing results from two trials with different methods of measuring the outcome can result in inconsistent conclusions about the treatment's efficacy between the two studies.

- ○ False
- ◉ True

10. Observational studies should only be pursued after an intervention has been proven to be effective in a randomized clinical trial.

- ○ True
- ◉ False