Common Study Designs in Clinical Research

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Learning Objectives

After this session, participants will be able to:

1. List three common study designs.

2. Discuss the advantages and disadvantages of case-control studies.

3. Discuss the advantages and disadvantages of cohort studies.
Case Report

17 year-old male.

No HIV infection.

No diabetes.

Hird 1994
Case Report

Next 3 days: vomiting, fever, swelling and pain.

4 days after symptom onset: admitted.
Case Report

Pulse of 155.
Blood pressure: 90/65.
Confused.
Creatine kinase (CK): 33,000 u/L.
AST 233.
ALT 63.
Annual Frequency in U.S. of Invasive Group A Streptococcal (GAS) Disease

• 23,650 cases of invasive GAS disease.

• About 2.4% of these cases were GAS toxic shock syndrome: 567.

• Source: CDC, Active Bacterial Core Surveillance (ABCs) Report, Group A Streptococcus, 2017.
If you have limited funds and time, what study design is appropriate for the investigation of risk factors for a rare condition such as GAS toxic shock syndrome?

A. Prospective cohort.
B. Case-control.
C. Clinical trial.
D. Case series.
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A. Prospective cohort.

B. Case-control.

C. Clinical trial.

D. Case series.
Branches of Epidemiology

- Descriptive.
- Analytic.

Hennekens & Buring, 1987
Descriptive Epidemiology

• Concerned with distribution of disease.

• Propose hypotheses.
Analytic Epidemiology

• Focuses on causes of diseases.

• Tests hypotheses.
Overview of Study Designs

• Descriptive
  • Individuals (case series).
  • Populations (correlational studies).

• Analytic
  • Observational studies.
  • Intervention studies/Experiments.
Observational studies

- Case-control:
  - Density case-control.
  - Cumulative case-control.
  - Case-cohort.

- Cohort (retrospective or prospective).
- Cross-sectional prevalence survey.
- Case-crossover.

Rothman, 2012
-- Intervention studies

• Nonrandomized controlled clinical trial.

• Randomized controlled clinical trial.
1. Systematic reviews, meta-analyses.
2. Randomized controlled trials with definitive results.
3. Randomized controlled trials with non-definitive results.
4. Cohort studies.
5. Case-control studies.
6. Cross sectional surveys.
7. Case reports.
True or False?

• Every research question in medicine and public health can be answered using a clinical trial.
True or False?

• Every research question in medicine and public health can be answered using a clinical trial.

FALSE
Basic Features of a Case-Control Study

• Subjects are selected by the presence or absence of the disease or outcome:
  – Cases have the disease.
  – Controls do not have the disease.

• **Cases** and **controls** are compared to one another with respect to their exposure(s).

Hennekens & Buring, *Epidemiology in Medicine*
Basic Features of a Case-Control Study

• Most are retrospective. That is, cases have already occurred.

• Whether or not it’s a retrospective case-control study or a prospective case-control study, you start with the outcome/disease and look back in time.
Advantages of the Case-Control Study Design

• Can study rare diseases.

• Multiple exposures.

• Usually cheaper than prospective cohort studies.
Disadvantages of the Case-Control Study Design

• No incidence (usually).

• Temporality may not be intact: did the suspected cause truly occur before the outcome?

• More prone to selection and recall bias than other designs.
Basic Features of a Cohort Study

• A study in which subjects are classified on the basis of the presence or absence of exposure to a suspected risk factor for a disease or other outcome.

Hennekens & Buring, *Epidemiology in Medicine*
Basic Features of a Cohort Study

• At the start of the study, all of the potential subjects must be free of the disease (the outcome).

• The two groups are compared to one another: risk or rate of the disease in the exposed vs. risk or rate of disease in the nonexposed (unexposed).
Two Types of Cohort Studies

• Retrospective.

• Prospective.
Retrospective Cohort

• Both the exposure and outcome have already occurred when the study is started.

• Start with the exposure.

• Follow subjects up until some point in the past.
Advantages of Cohort Studies

• Incidence.

• Temporality (time sequence).

• Multiple outcomes.

• Valuable for studying rare exposures.
Disadvantages of Cohort Studies

• If prospective, can be very expensive and time consuming.

• If retrospective, requires the availability of adequate records.

• Validity can be affected by losses to follow-up.

• Usually not efficient for the study of rare diseases.
Key to the next 3 slides (adapted from textbook *Epidemiology in Medicine* by Hennekens & Buring)

- = Present
- = Absent
? = To be determined

Basis on which groups are selected at start of study

The investigator at the beginning of the study
Prospective Cohort Study

Exposure  ?

Disease  ?
Retrospective Cohort Study

Exposure

Disease

??
Case-Control Study

Exposure

Disease

?  

?
Conclusions

• Case-control studies, cohort studies, and clinical trials are common in clinical research.

• Each study design has its strengths and limitations.
Cited References


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Think (5 minutes): What research question would you like to pose in the next few months? What’s the best study design?

Pair (10 minutes): Find a partner and strategize.

Share (15 minutes).