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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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.

Analytic Study Designs

- **Observational studies**
  - Case-control:
    - Density case-control.
    - Cumulative case-control.
    - Case-cohort.
  - Cohort (retrospective or prospective).
  - Cross-sectional prevalence survey.
  - Case-crossover.

Rothman, 2012

Analytic Study Designs

–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.

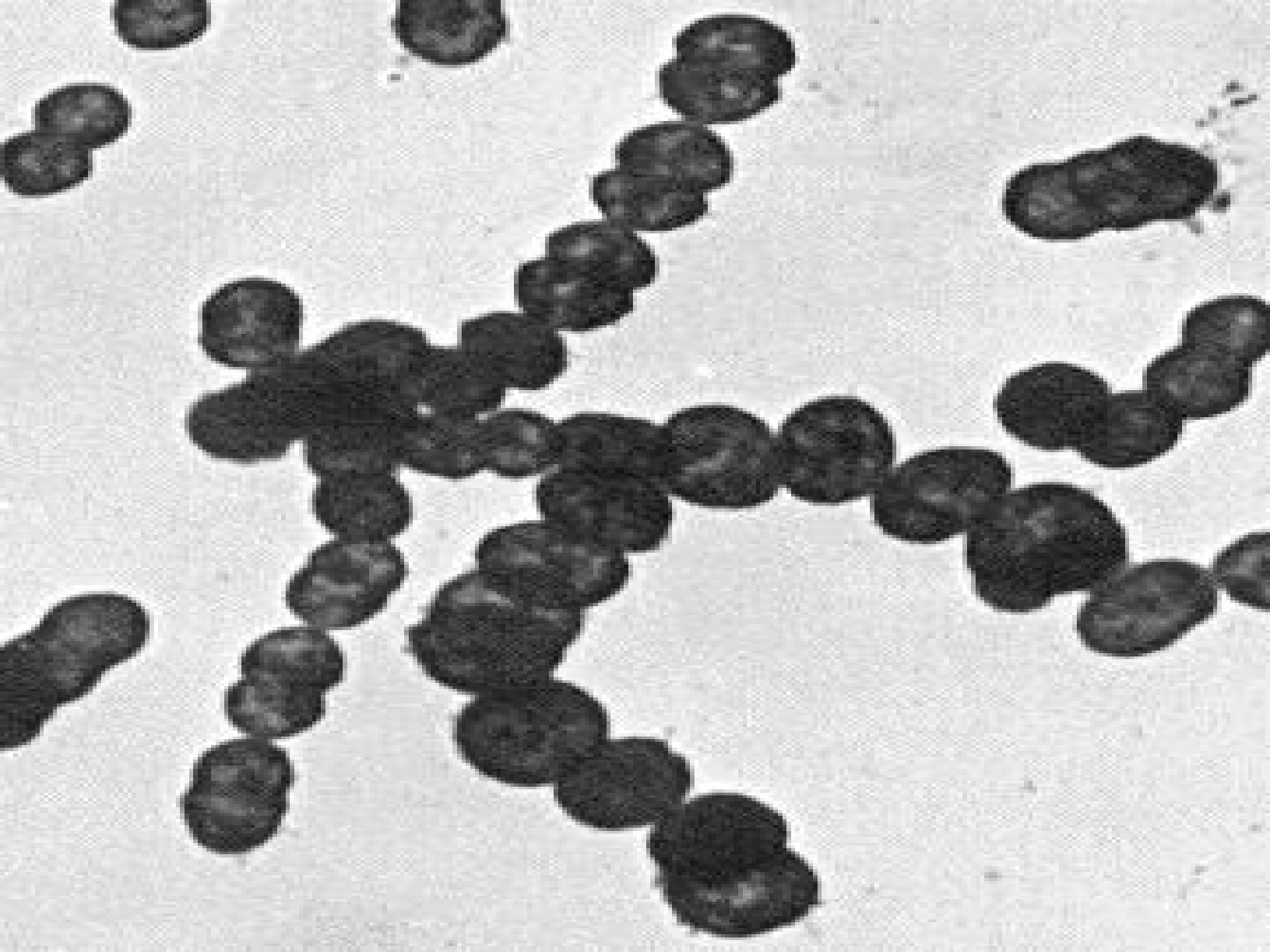
True or False?

- 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

} *Basis on which groups are selected at <u>start</u> of study*

? = To be determined

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 → **Pair** → **Share**

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).