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Overview of studies

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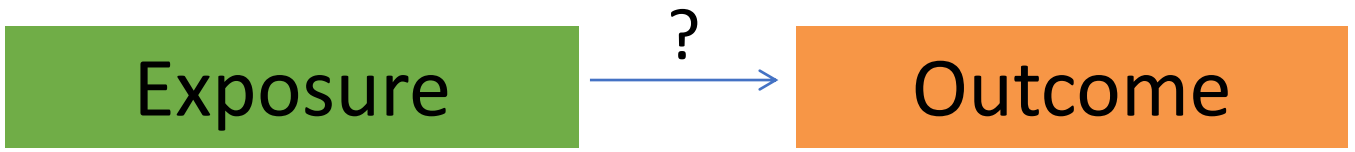
Descriptive vs. analytic studies

Descriptive study	Analytic study
• No advance hypothesis • Accept that associations may or may not be causal • Often use pre-existing data	• Driven by hypothesis or hypotheses • Hypothesis usually proposes a causal link • More often require new data collection

Hypothesis for analytic study



Synonyms:

- Risk factor
- Possible cause
- Predictor
- Independent variable

- Disease
- Effect
- Response
- Dependent variable

Experimental vs. observational studies

Experimental study	Observational study
• Investigator assigns exposure status • More closely resemble controlled laboratory experiments • “Gold standard” of epidemiology • Not feasible or ethical for some exposures	• Investigator observes exposure status • More feasible and ethical for some exposures

Experimental studies

- Purpose: Investigate the role of some agent in the prevention or treatment of disease
  - Preventive or prophylactic trial
  - Therapeutic or clinical trial
- Investigator assigns individuals to:
  - Treatment group(s)
  - Comparison group(s) (e.g., placebo)
- Usually using process of randomization
- Selecting study participants
  - Eligibility criteria
    - Prevention trial: healthy or high-risk individuals
    - Clinical trial: individuals with specific diseases
  - Informed consent

Experimental studies

- Selecting study participants
  - Eligibility criteria
    - Prevention trial: healthy or high-risk individuals
    - Clinical trial: individuals with specific diseases
  - Informed consent
- Random assignment
  - Groups are similar to each other on other factors
  - Equipoise is necessary
    - Uncertainty about best course of action
- Analysis
  - Outcomes compared in treatment and comparison groups
    - Intent-to-treat analysis
      - Groups are analyzed according to randomization regardless of actual compliance
    - Efficacy analysis
      - Groups are analyzed according to compliance

Experimental studies

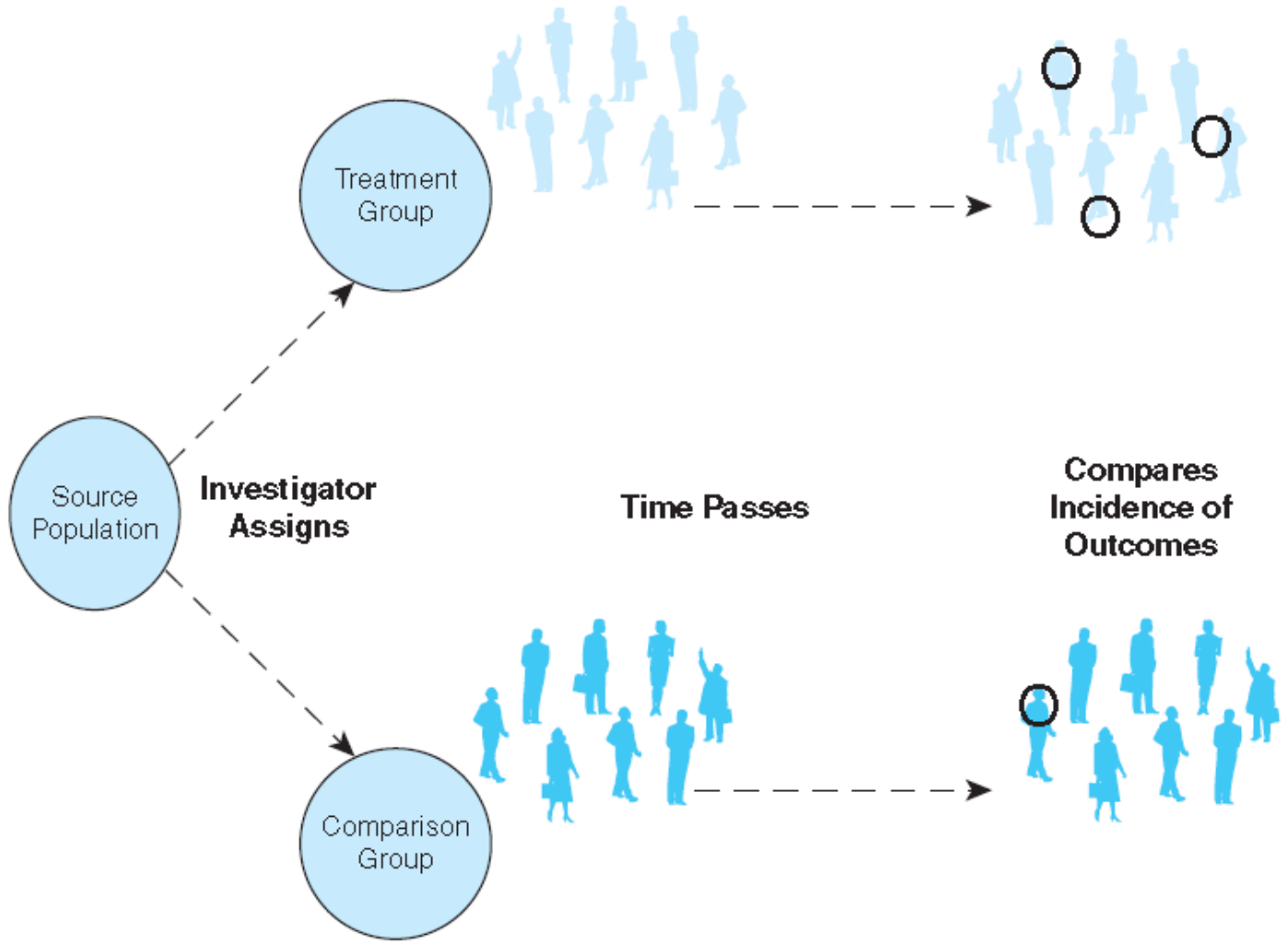


FIGURE 6–1 Schematic Representation of Experimental Study Implementation

Experimental studies

Strengths	Weaknesses
• Superior control over confounding factors, even if unknown or hard to measure • Exposure clearly precedes outcome • Can estimate incidence in both groups • Easy to study several outcomes	• Not always possible or ethical to manipulate exposure at random • Inefficient for rare or long-delayed outcomes

Observational analytic studies

- Cohort studies
  - Healthy subjects are selected according to their exposure status and followed over time to determine the incidence of disease
- Case-control studies
  - Subjects are selected according to their disease status and their exposure histories are reviewed

Cohort studies

- Purpose: Investigate the causal or preventive role of a particular exposure
- Cohort = group of people with common characteristic
- Also known as follow-up, incidence, or longitudinal studies

Cohort studies

- Selecting study participants
  - Special cohorts for rare exposures
  - General population cohorts for more common exposures
  - “Exposed” and “Unexposed” groups
  - Relative sizes of exposed and unexposed groups need not reflect frequency of exposure in underlying population

Cohort studies

- Prospective cohort study
  - Individuals grouped based on past or current exposure and followed into future to observe outcomes
  - Outcome has not yet occurred at the start of the study
- Retrospective cohort study
  - Both exposures and outcomes have already occurred at the start of the study

Cohort studies

- Analysis
  - Cumulative incidence or incidence rate compared in exposed and unexposed groups
  - Multiple outcomes can be assessed

Cohort studies

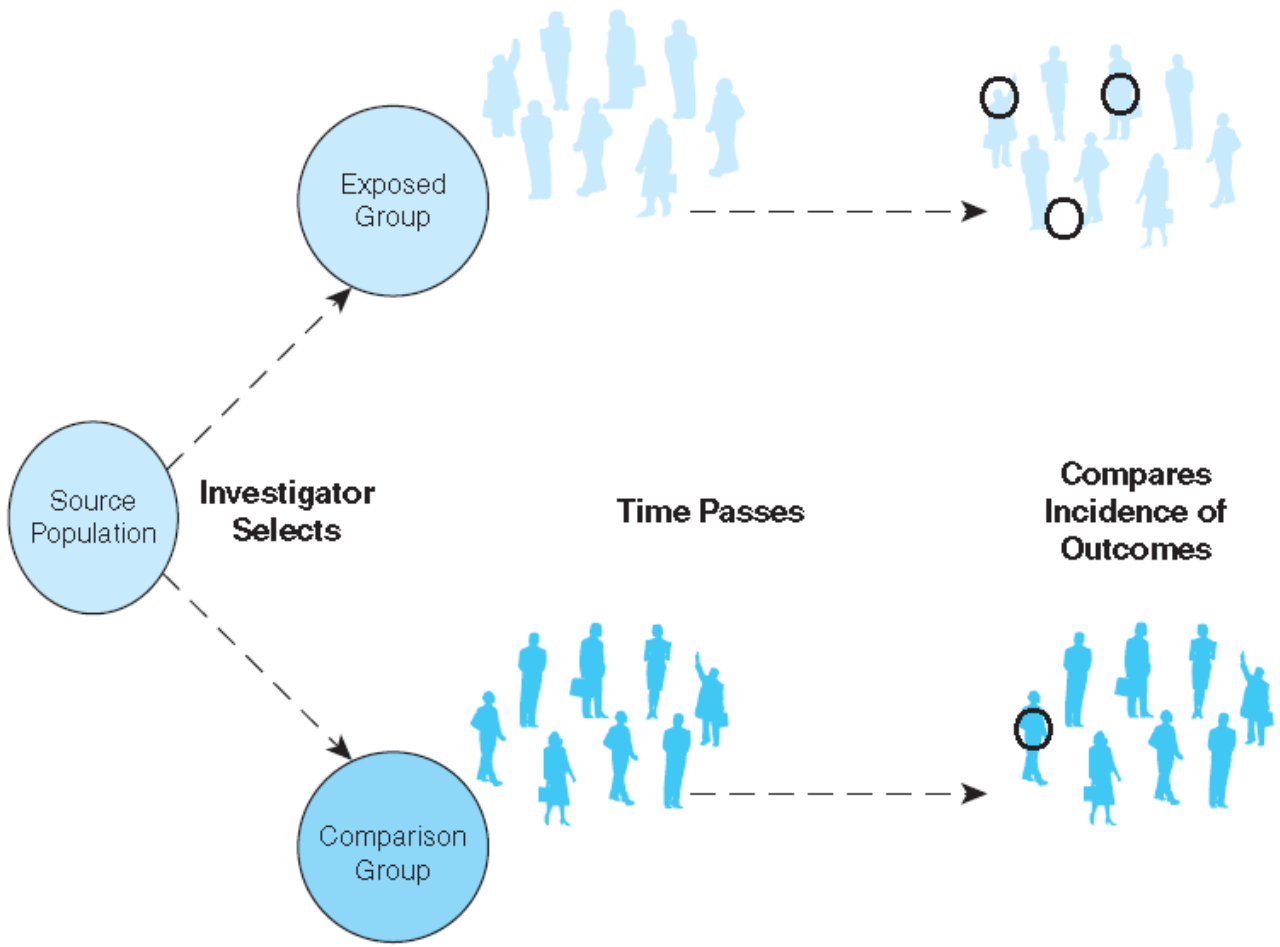


FIGURE 6–2 Schematic Representation of Cohort Study Implementation

Cohort studies

Strengths	Weaknesses
• Exposure known to precede outcome • Can estimate incidence in both groups • Easy to study multiple outcomes • Efficient for rare exposures	• Inefficient for rare outcomes • If prospective, can be costly for large samples or delayed outcomes

Cohort (A) or Case-control study (B)

- Interested in rare outcome?
- Interested in rare exposure?
- Interested in multiple outcomes?
- Interested in multiple exposures?
- Interested in outcome that takes a long time to develop?

Descriptive studies

- Cross-sectional studies
- Ecologic studies

Cross-sectional studies

- Purpose: Examine associations between diseases and other variables of interest in a defined population at one particular time
- Snapshot of population at one time
  - Measure disease prevalence in relation to exposure prevalence
  - Cannot determine if exposure preceded disease

Ecologic studies

- Purpose: Examine rates of disease in relation to population-level exposure measures
  - Units of analysis are groups rather than individuals
- Associations observed at the group level do not necessarily hold at the individual level
- Usually quick and inexpensive when using available data

Choice of study design

- Research question
- Existing scientific knowledge
- Frequency of exposure and disease
- Ethical considerations
- Concerns about validity, random error, efficiency

Choice of study design

- Can use multiple study designs to assess one particular research question
- Example: We hypothesize that people who consume a diet high in vitamin A have a lower risk of lung cancer as compared to people who consume a diet low in vitamin A.

Ecologic study

- Relate mean vitamin A consumption at the state level to state-level lung cancer mortality rates

Cross-sectional study

- Survey subset of particular population
- Ascertain information about vitamin A consumption
- Ascertain information about lung cancer status

Case-control study

- Select lung cancer cases
- Identify suitable controls
- Ascertain past vitamin A consumption among cases and controls

Cohort study

- Select individuals with high and low levels of vitamin A consumption
- Follow over time for development of lung cancer
- Compare incidence of lung cancer in those with high and low levels of vitamin A consumption

Experimental study

- Assign individuals to high and low levels of vitamin A consumption
- Follow over time for development of lung cancer
- Compare incidence of lung cancer in those with high and low levels of vitamin A consumption