Project: Ghana Emergency Medicine Collaborative

Document Title: Research Design

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Research Design

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Health research is
the process of systematically
investigating
a single well-defined aspect of
physical, mental, or social well-being.

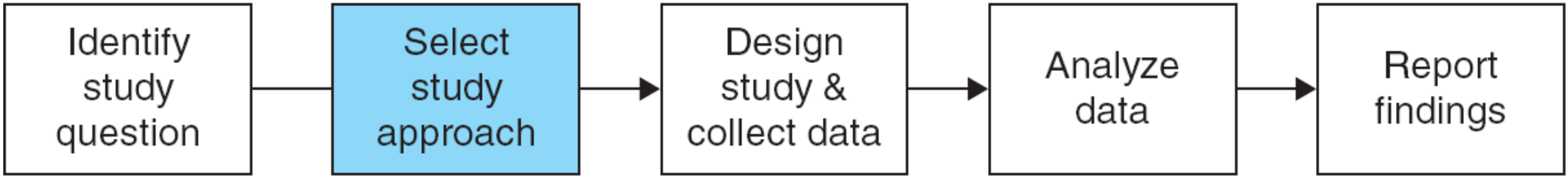
Examples of Health Research

- Is an 8-week physical therapy program effective at reducing the risk of anterior cruciate ligament tears in high school athletes?
- What are the most common signs and symptoms associated with multiple sclerosis?
- How much does the risk of severe hearing loss increase with age?
- Which factors predict binge drinking behavior in college and university students?

Examples of Research Goals

- Identifying and classifying new health problems
- Determining risk factors for disease
- Developing and testing new interventions for preventing or treating illness
- Evaluating the impact of health policies on health outcomes
- Synthesizing existing knowledge so that it can be applied by others

Research Process



Types of Study Designs

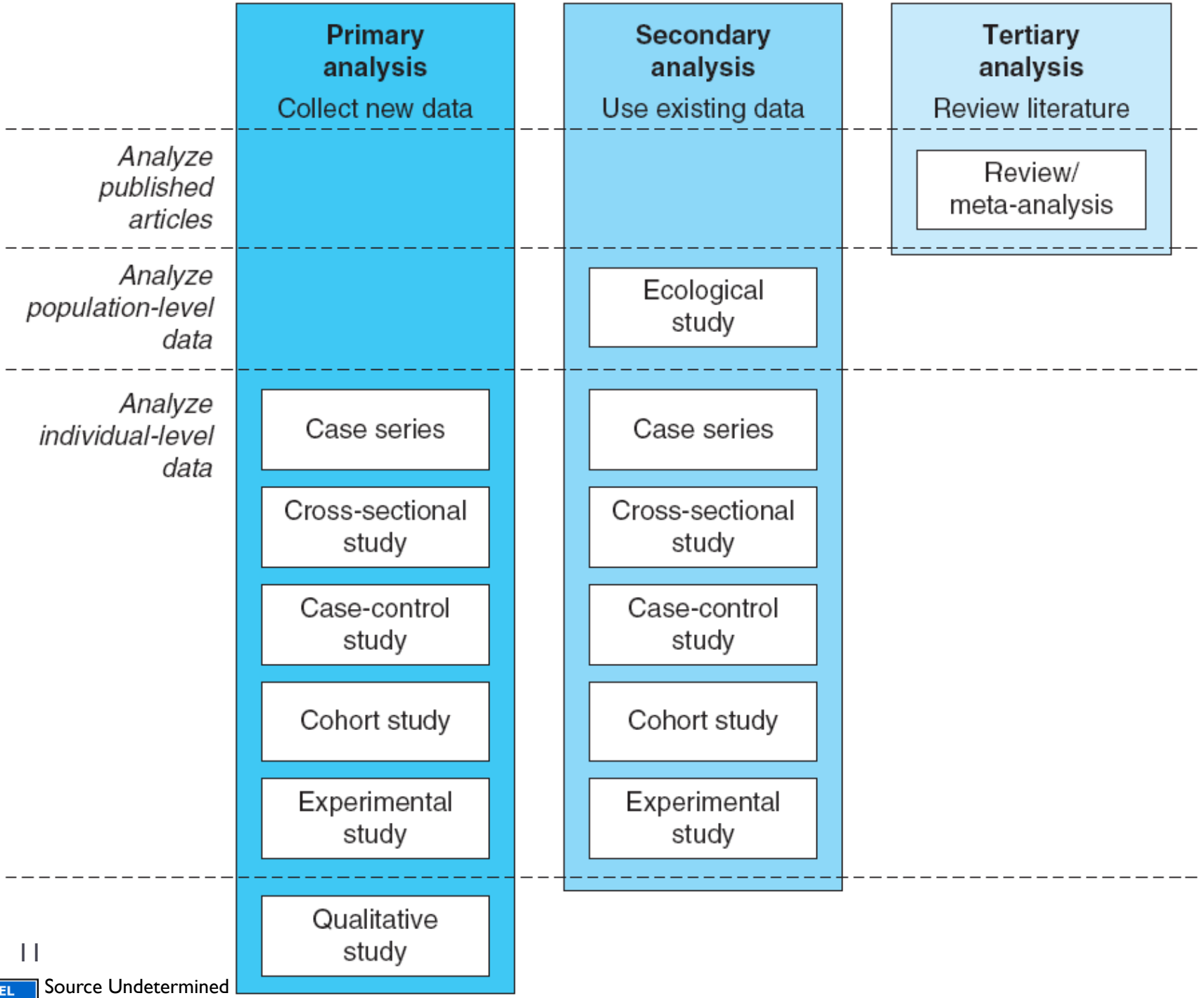
- Reviews & meta-analyses
- Correlational (ecological)
- Case series
- Cross-sectional
- Case control
- Cohort
- Experimental & quasi-experimental
- Qualitative

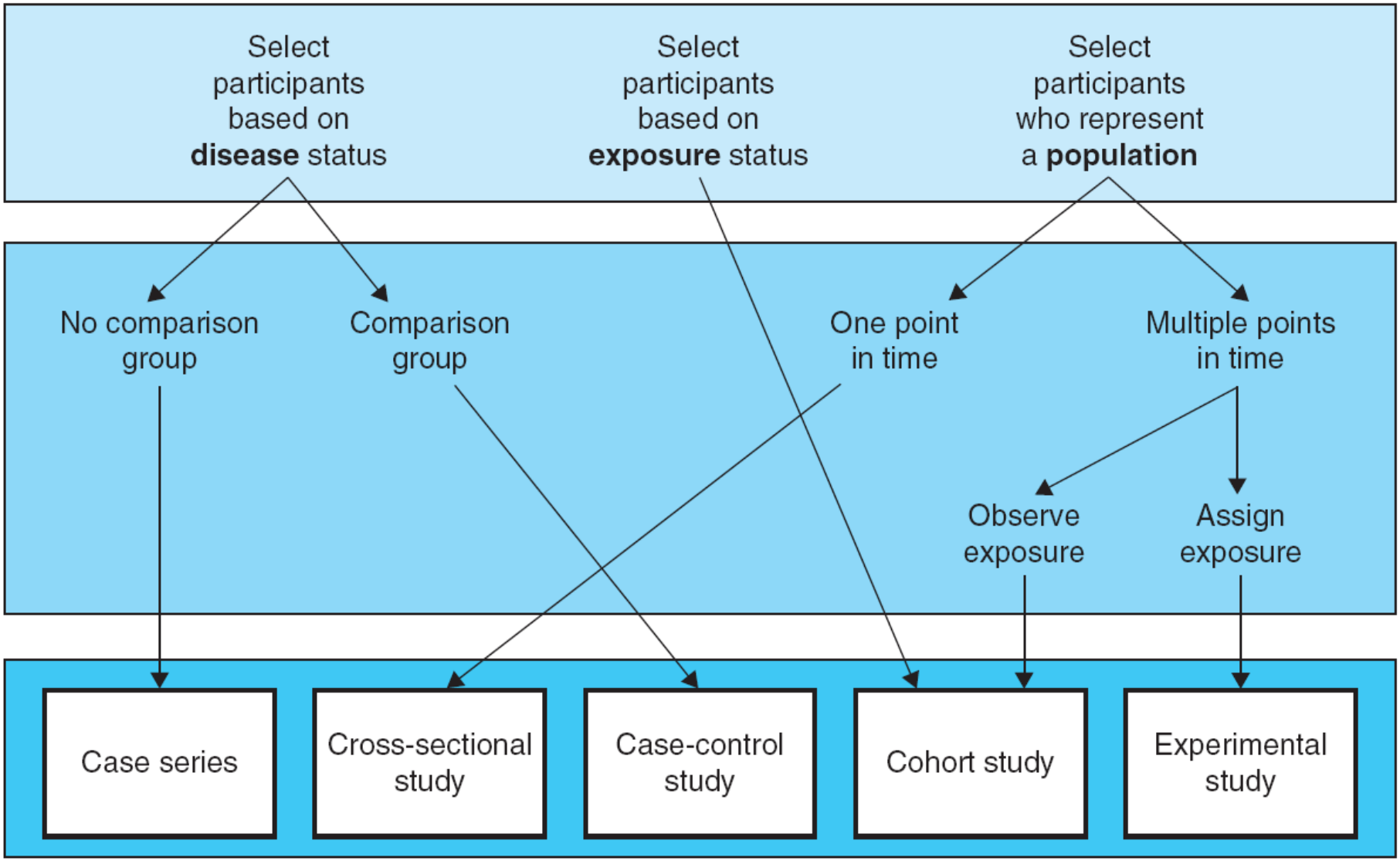
Epidemiology Terms

- Prevalence: Amount of a disease in a population at a point in time. Unit-less data.
  - Example: Prevalence of HIV in Ghana is X%.
- Incidence: Number of new cases in a defined period of time.
  - Example: Incidence of HTN in 1999 was 150 cases per 100,000 persons (not actual data).
- Rate Ratio: Rate in the exposed/rate in unexposed
  - Calculated in cohort studies
- Odds Ratio: Approximates the rate ratio
  - Calculated in case-control studies
- 95% Confidence Interval: There is a 95% change that the true value measured falls within the boundaries.
  - Can not include 1.0. If it does it is not significant
- P value: measure the *statistical* significance of a study is less than 0.05

Goals of types of studies

Study Approach	Goal
Review/meta-analysis	Synthesize existing knowledge
Correlational (ecological) study	Compare average levels of exposure and disease in several populations
Case series	Describe a group of individuals with a disease
Cross-sectional survey	Describe exposure and/or disease status in a population
Case-control study	Compare exposure histories in people with disease (cases) and people without diseases (controls)
Cohort study	Compare rates of new (incident) disease in people with different exposure histories or follow a population forward in time to look for incident diseases
Experimental study	Compare outcomes in participants assigned to an intervention or control group
Qualitative study	Seek to understand how individuals and communities perceive and make sense of the world and their experiences





Study Design: Reviews & Meta-Analyses

- Carefully gathers all prior publications on a specific topic and summarizes them to provide a big-picture analysis
  - Systematic reviews attempt to locate ALL the articles on a well-defined topic
    - Key words
    - Inclusion criteria
  - Meta-analysis combines the results of several high-quality articles that used similar methods to collect and analyze data into one summary statistic

Analysis

- Systematic Review – summary table of studies and findings
- Meta-analysis

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www.healthstrategy.com/
meta/meta/.pl

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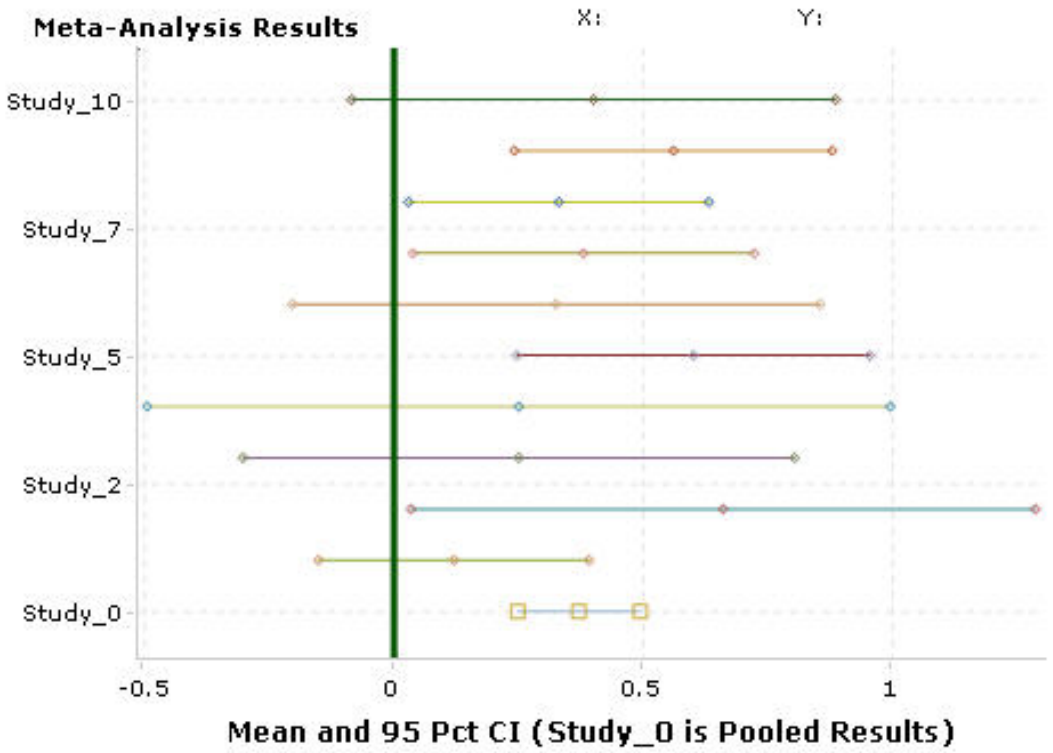
www.healthstrategy.com/
meta/meta-analysis.jpg



Study Design: Ecological

- AKA: cross-sectional, correlational, aggregate
- These studies use **population-level data** to examine the relationship between exposure rates and disease rates
  - Associations
  - Does the percentage of adults with multiple sclerosis tend to be higher in countries farther from the equator?

Analysis

Correlation, linear regression, rate adjustment

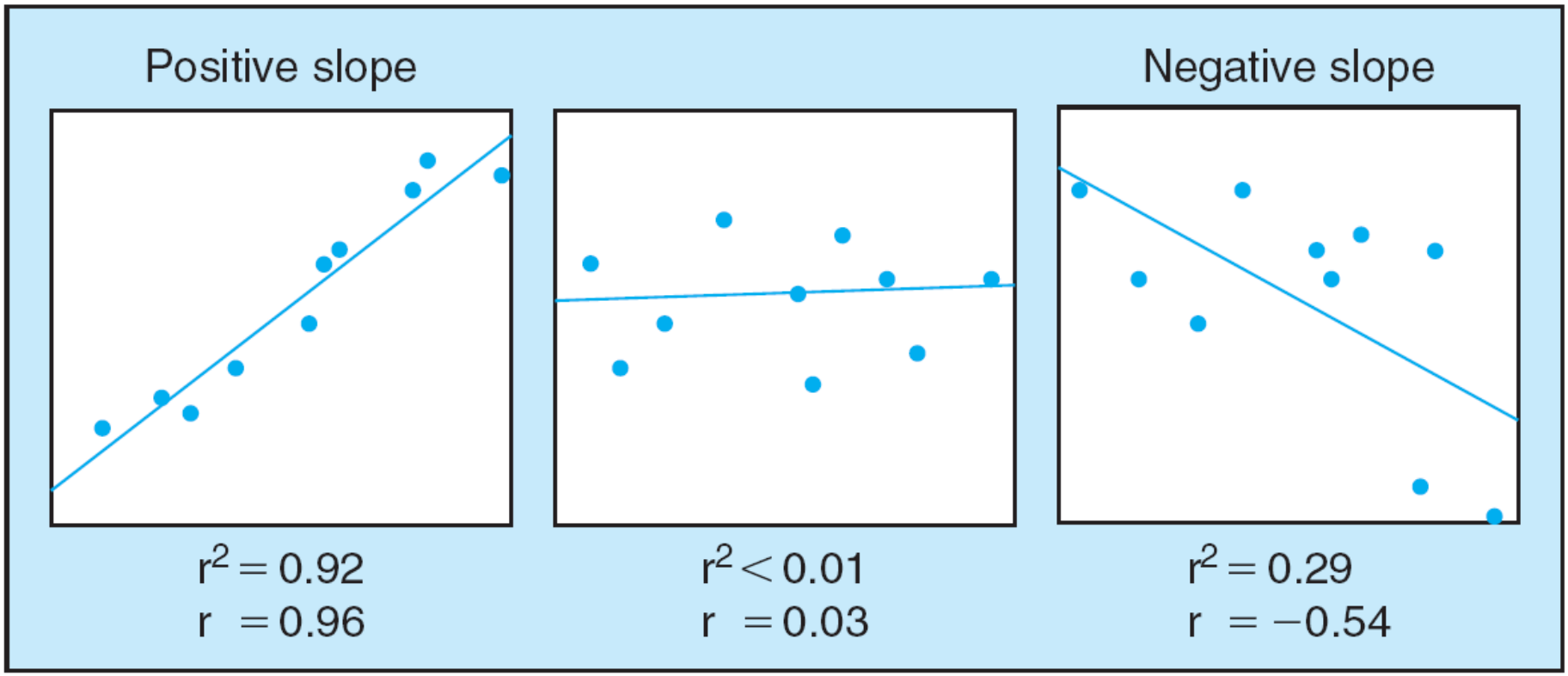


FIGURE 8-3 Correlation

Objective	Compare average levels of exposure and disease in several populations
Primary study question	Do populations with a higher rate of exposure have a higher rate of disease?
Population	Existing population-level data are used; there are no individual participants.
When to use this approach	The aim is to explore possible associations between an exposure and a disease using population-level data.
Requirement	The topic has not been previously explored using individual-level data.
First steps	1. Select the sources of data that will be used. 2. Decide on the variables to include in the analysis.
What to watch out for	The ecological fallacy
	Limited publication venues
Key statistical measure	Correlation

Study Design: Case Studies

- Case report – 1 patient
- Cases series – 2 or more patients with the same disease or the same procedure
  - Describe characteristics or similarities
  - Identify new syndromes; refine case definitions
  - Clarify typical disease progression
  - Develop hypotheses for future research
  - THINK: person, place, time

Analysis

- Descriptive statistics
  - Numeric summary (mean, median, mode)
  - Count or frequency

Objective	Describe a group of individuals with a disease
Primary study question	What are the key characteristics of the cases in this study population?
Population	All individuals in the study must have the same disease or be undergoing the same procedure.
When to use this approach	A source of cases is available, and no comparison group is required or available.
Requirement	An appropriate source of cases is available.
First steps	1. Specify what new and important information the analysis will provide. 2. Identify a source of cases. 3. Assign a case definition. 4. Decide on the characteristics of the study population that will be described.
What to watch out for	A lack of generalizability
Key statistical measure	Only descriptive statistics are required.

Study Design: Prevalence

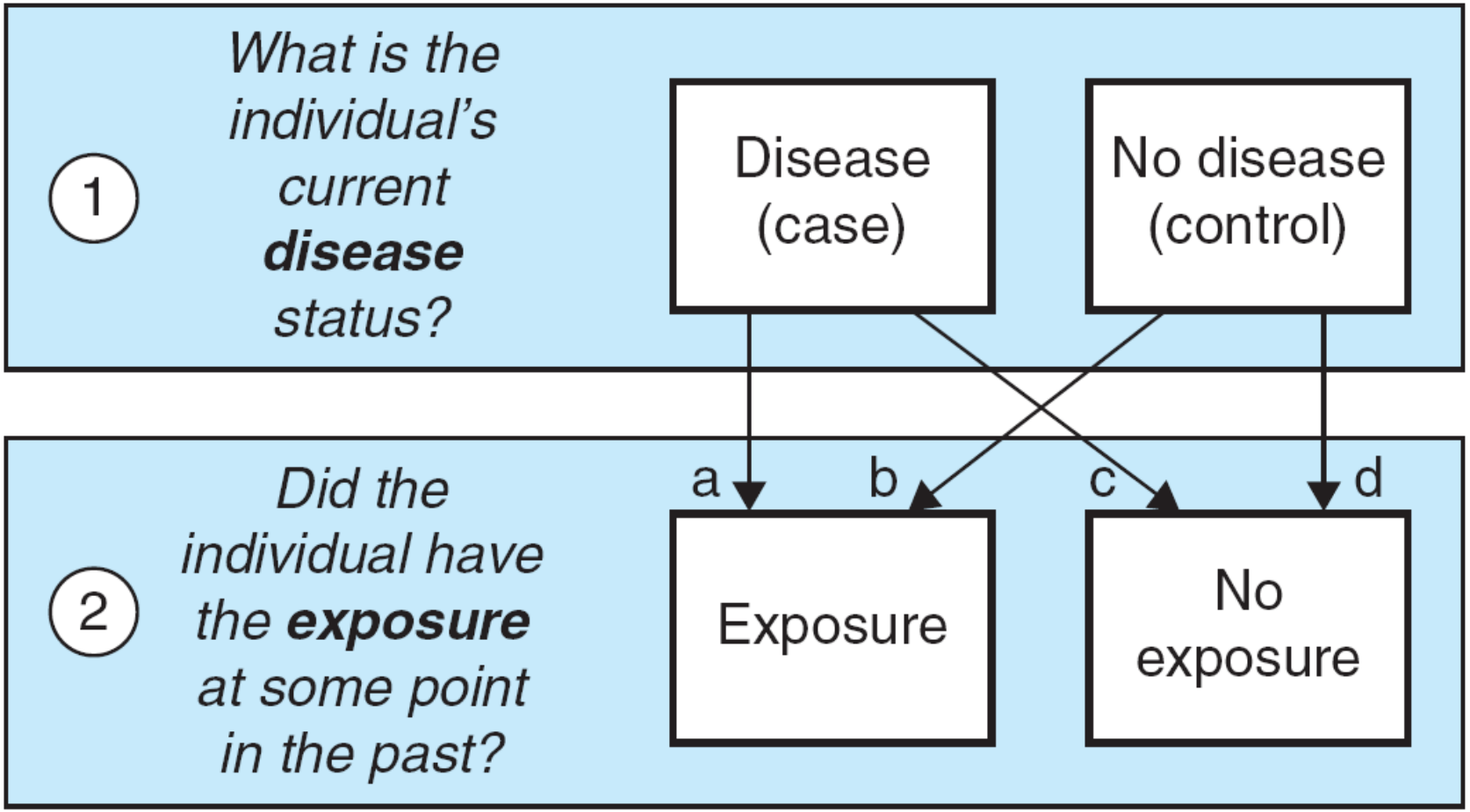
- AKA cross-sectional
- Measures the proportion of a population with a particular exposure or disease at one point in time, based on a representative sample
  - Survey or questionnaire

Analysis

- Prevalence
- Prevalence ratios (association)

Objective	Describe the exposure and/or disease status in a population
Primary study question	What is the prevalence of the exposure and/or disease in the population?
Population	The study participants must be representative of the population from which they were drawn.
When to use this approach	Time is limited and/or the budget is small.
Requirement	The exposures and outcomes are relatively common, and the likelihood of being able to recruit several hundred participants is strong.
First steps	1. Define a source population. 2. Develop a strategy for recruiting a representative sample. 3. Decide on the methods to be used for data collection.
What to watch out for	Nonrepresentativeness of the study population
Key statistical measure	Prevalence

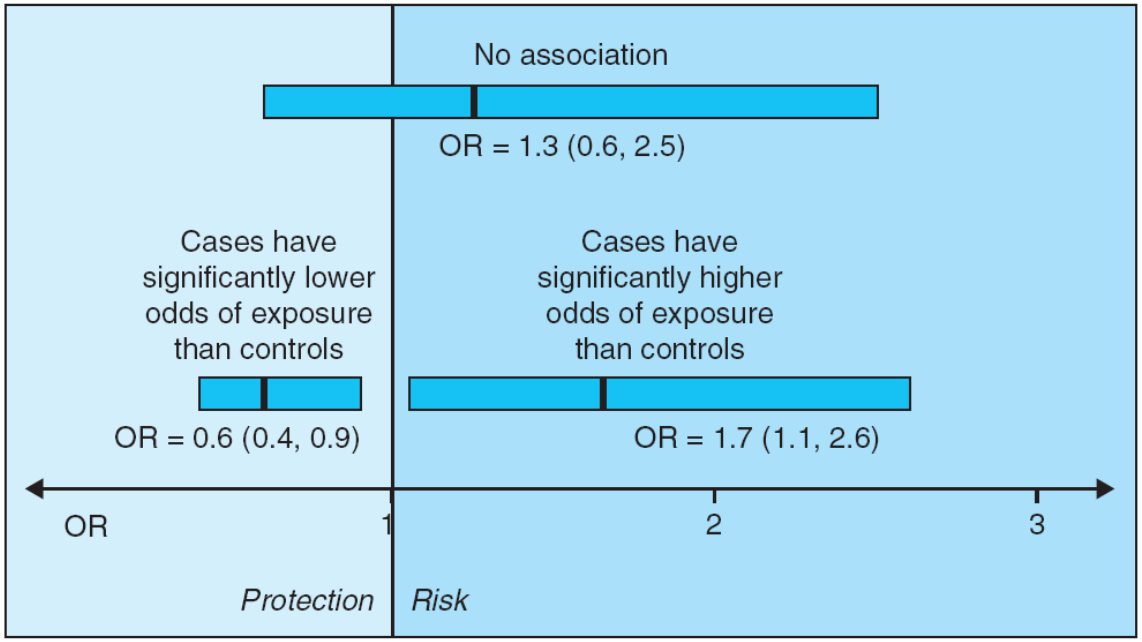
Study Design: Case-Control



- Efficient for rare diseases

Analysis

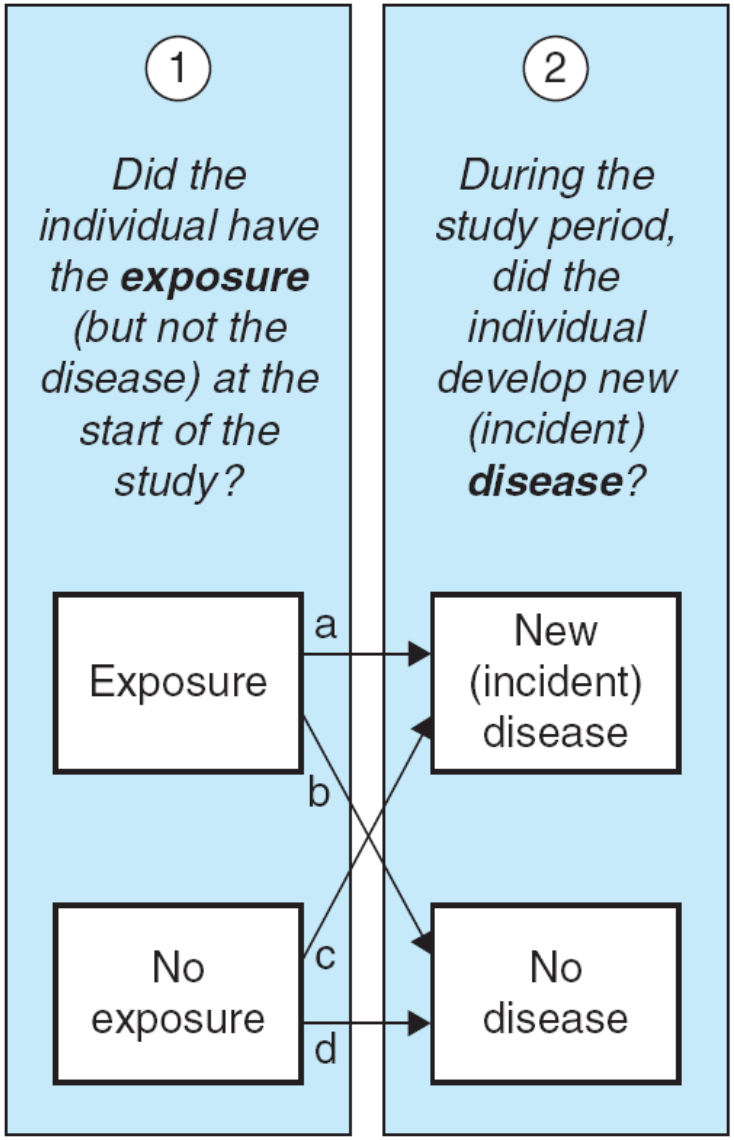
- Odds, odds ratio, 95% confidence interval
- Matched odds ratio (individual matching)
- Sensitivity analysis to assess potential effect of bias
  - Misclassification
  - Recall



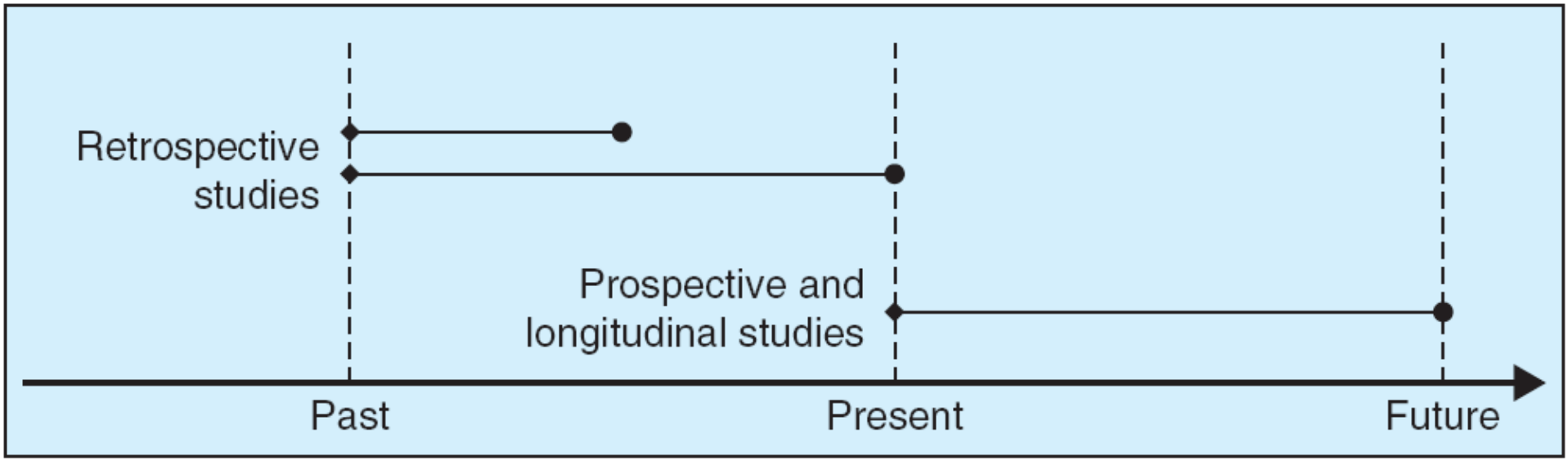
Objective	Compare exposure histories in people with disease (cases) and people without diseases (controls)
Primary study question	Do cases and controls have different exposure histories?
Population	Cases and controls must be similar except for their disease status.
When to use this approach	The disease is relatively uncommon, but a source of cases is available.
Requirement	A source of cases is available.
First steps	1. Identify a source of cases. 2. Assign a case definition. 3. Decide what type of control population will be appropriate for the study. 4. Decide whether cases and controls will be matched.
What to watch out for	Recall bias
Key statistical measure	Odds ratio (OR)

Study Design: Cohort

- Follows participants through time to calculate the rate of incident disease & identify risk factors
  - Baseline measurement
  - Follow-up measurement(s)



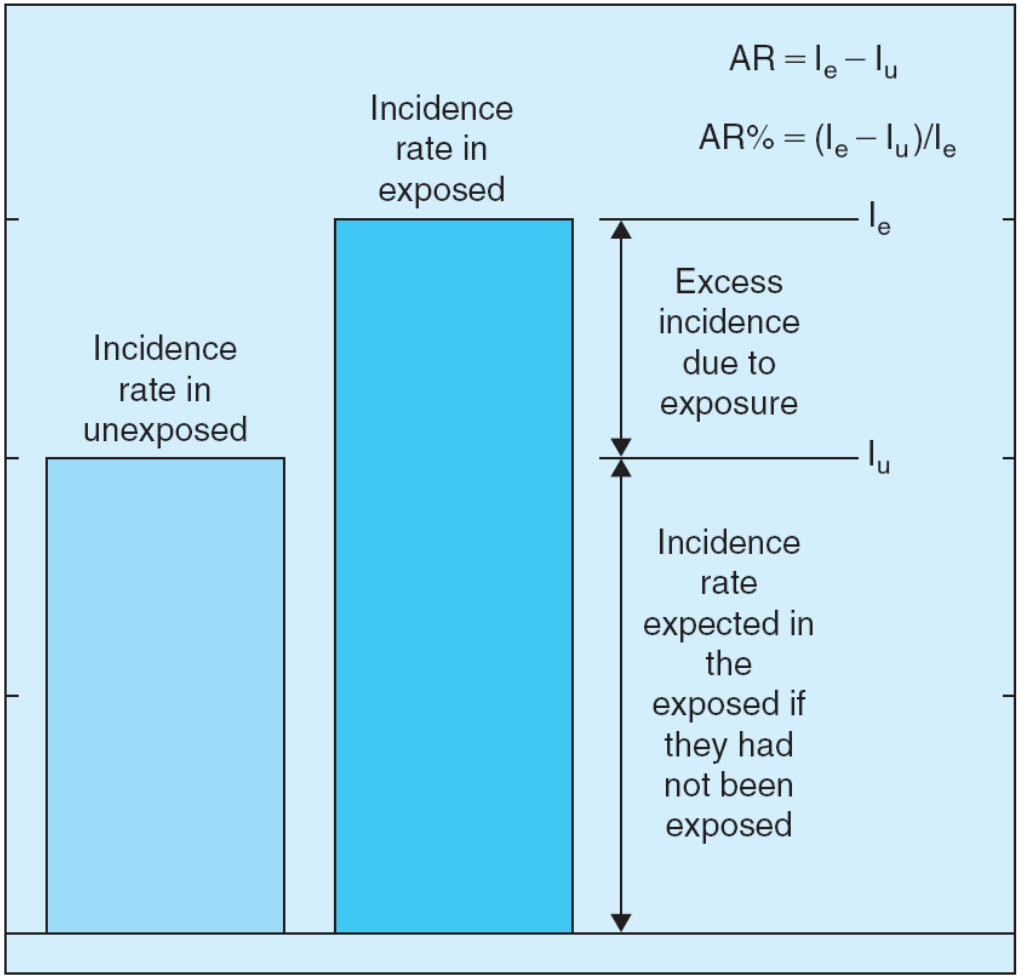
Thinking about time



A longitudinal cohort recruits participants based on their membership in a well-defined source population (prospective studies don't necessarily rely on source populations)

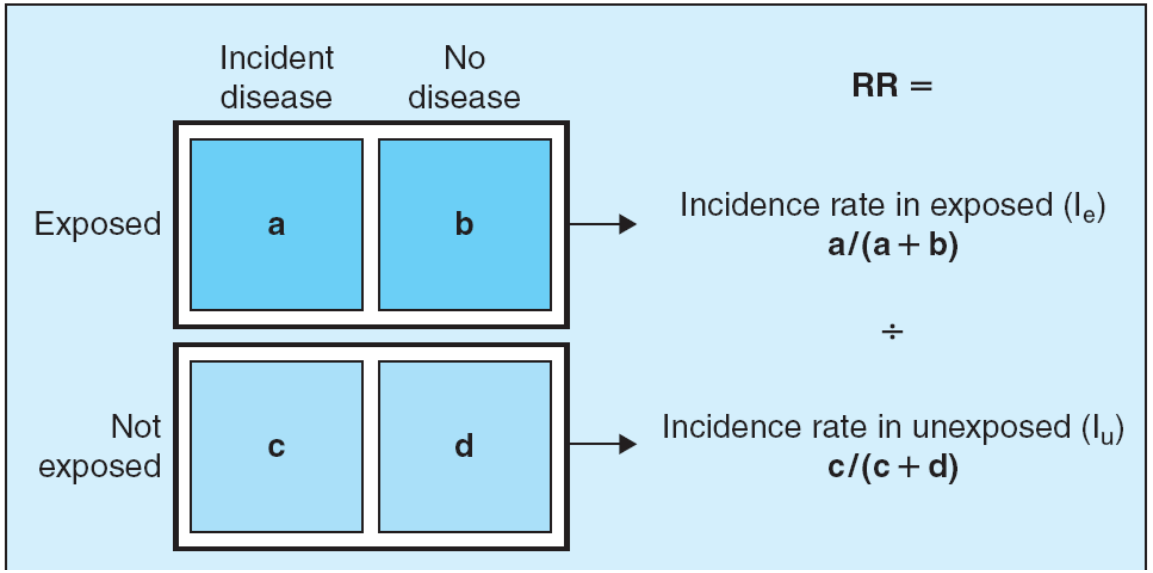
Analysis

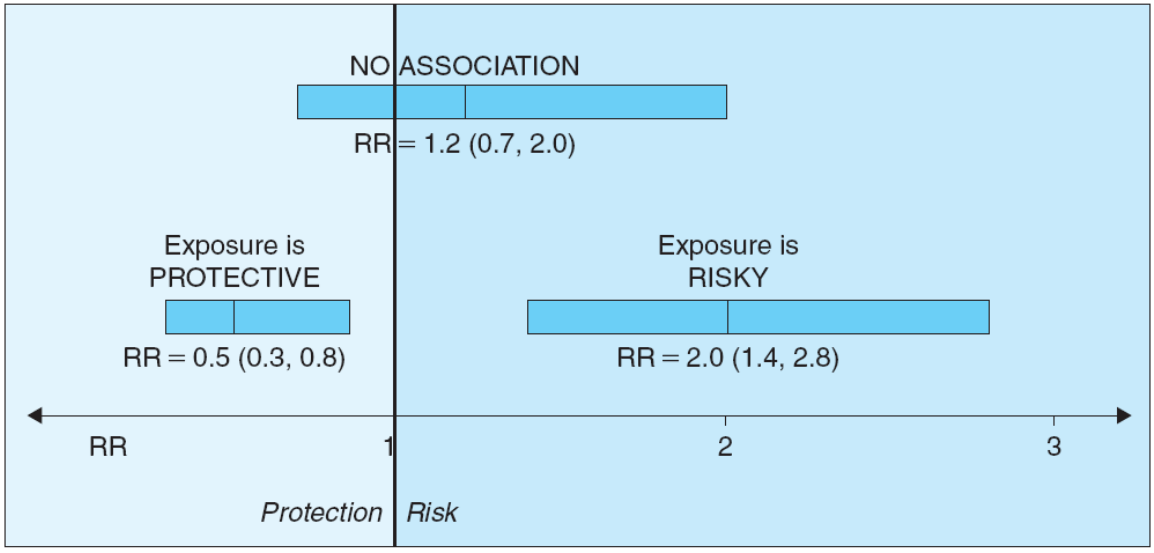
- Incidence rate
- Attributable risk – absolute difference in incidence rate between exposed & unexposed
  - Attributable risk % (attributable fraction among the exposed)



Analysis, cont.

- Rate ratio & 95% confidence interval
  - AKA: relative rate, risk ratio, relative risk





Approach	Prospective or Retrospective Cohort	Longitudinal Cohort
Objective	Compare rates of new (incident) disease in people with different exposure histories.	Follow a population forward in time to look for new (incident) diseases.
Primary study question	Is exposure associated with an increased incidence of disease?	Is exposure associated with an increased incidence of disease?
Population	Participants must be similar except for exposure status.	Participants must be available for follow-up months or years after enrollment.
	Because the goal is to look for incident disease, no one can have the disease of interest at the start of the study.	The study participants must be reasonably representative of the population from which they were drawn.
When to use this approach	An exposure is relatively uncommon, but a source of exposed individuals is available.	The goal is to examine multiple exposures and multiple outcomes, and time is not a concern.
Requirements	A source of individuals with the exposure is available.	There is adequate time and money for the study.

First steps	1. Identify a source of individuals with the exposure. 2. Decide what type of unexposed individuals will be an appropriate comparison group.	1. Select a source population. 2. Select the exposures and outcomes that will be assessed. 3. Decide how often data will be collected. 4. Develop a strategy for minimizing the burden of participation and maximizing benefits and incentives.
What to watch out for	Participant drop-outs (prospective studies) or missing records (retrospective studies)	Participant drop-outs
	Information bias, in which the exposed participants are more thoroughly examined for disease than unexposed participants	Potential data management challenges if lots of information is collected at many points in time.
Key statistical measure	Relative risk (rate ratio, RR)	Relative risk (rate ratio, RR)

Study Design: Qualitative Methods

- Look for themes and meanings emerging from observation on a small, selected group of informants
  - Phenomenology – how do pt understand/interpret
  - Grounded theory – develop general theories based on observation
  - Ethnography – insiders view of a group's perspective

Qualitative, cont.

- In-depth and semi-structured interviews
  - Open-ended questions (probing is allowed)
  - Possibly supplemented (diary, journal)
- Focus group
  - 4-12 people
  - Moderator led discussion

Analysis

- Coding/classifying observations
  - Derive major & minor themes
  - Quotations often used
  - Possibly minimal use of descriptive statistics (counts)

Example

CIGARETTE SMOKING AND RENOVASCULAR HYPERTENSION
The Lancet, Volume 322, Issue 8353, Pages 765 - 766, 1 October 1983

- A retrospective cohort study to investigate the association between smoking and renal artery stenosis compared 71 patients with documented renovascular hypertension and 308 age-matched control patients with essential hypertension. 94% (30/32) of men and 74% (29/39) of women with renal artery stenosis had smoked cigarettes compared with only 43% (64/150) of men and 41% (65/158) of women in the control group. This striking relation was true for both patients with fibromuscular disease (71% smokers; 15/21) and patients with atherosclerotic lesions (88% smokers; 44/50). All renal artery stenosis groups had significantly higher systolic and diastolic blood pressures than the relevant control group. When the groups were stratified according to blood pressure, there were significantly more smokers in the renal artery stenosis group at every level of blood pressure.

Answers

Population?	71 patients with renovascular htn
Control Group?	Control: 308 age-matched controls
Disease?	Disease: renal artery stenosis
Exposure?	Exposure: smoking
Time?	Identify cases first, then found age-matched controls
Type of study?	Case control study – select the cases (disease) first then look back to exposure

Example

Leukaemia incidence after iodine-131 exposure. Lancet 1992 Jul 4;340(8810):1-4

Leukemia is one of the most prominent late effects of exposure to ionizing radiation. We have studied the incidence of leukemia among 46,988 Swedish patients exposed to iodine-131 (1311) for diagnostic reasons or to treat hyperthyroidism or thyroid cancer. The observed number of leukemia's was compared with that expected based on incidence data from the general population. The mean absorbed dose to the bone marrow was estimated as 14 mGy (range 0.01-2.226). 195 leukemia's occurred more than 2 years after exposure, and the standardized incidence ratio (SIR) was 1.09 (95% confidence interval 0.94-1.25). Similar, but again not significantly, increased risks were seen for chronic lymphocytic leukemia (CLL) (SIR = 1.08), a malignant condition not found to be increased after irradiation, and for non-CLL (SIR = 1.09). The risk of leukemia did not vary by sex, age, time, or radiation dose from 1311…

Answers

- Population?
- Control Group?
- Disease?
- Exposure?
- Time?
- Type of study?

- 46,998 Swedish patients exposed to iodine-131
- General population incidence rates
- Leukemia
- Iodine-131
- First identified exposure the followed and looked for disease
- Cohort study

Resources List

- Introduction to Health Research Methods – A Practical Guide. Kathryn H. Jacobsen. Jones and Bartlett Learning. 2012.