

Introduction to epidemiology and study designs

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What is epidemiology?

- Epidemiology is the study of factors affecting the health and illness of populations (or how often diseases occur in different groups of people and why)
- Discovery & examination of causal relationships
- It serves as the foundation to plan and evaluate strategies to prevent illness

Epidemiology 101

Today's Random Medical News

from the New England
Journal of
Panic-Inducing
Gobbledygook



Jim Borgman
The Cincinnati Enquirer
King Features Syndicate

Bradford-Hill criteria:

Assessing evidence of causation

- Strength of the association: the larger the association, the more likely that it is causal
- Consistency: Consistent findings observed by different persons in different places with different samples
- Specificity: Causation is likely if a very specific population at a specific site and disease with no other likely explanation
- Temporality: The effect has to occur after the cause

Bradford-Hill criteria:

Assessing evidence of causation

- Biological gradient: Greater exposure should generally lead to greater effect
- Plausibility: A plausible mechanism between cause and effect
- Coherence: Coherence between epidemiological and laboratory findings
- Analogy: The effect of similar factors may be considered

Key assumptions

- Disease doesn't occur at random
 - Epidemiology uses systematic approach to study the differences in disease distribution
- Disease has causal and preventive factors
 - These factors can be identified and strategies to prevent/delay onset of disease implemented and evaluated

Components of epidemiology

- Measure of disease frequency
- Distribution of disease
- Determinants of disease

Measuring disease frequency

- Frequency measures
 - Risk
 - Rate
 - Proportion
 - Odds
 - Prevalence
 - Incidence
- Chosen based on the type of study and the goal of the study

Measuring disease frequency

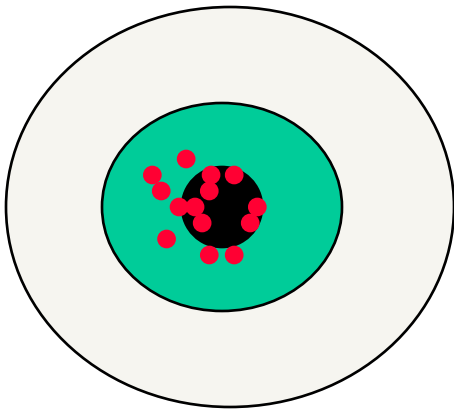
- Prevalence - the number of people with a particular condition at a specified time within a defined population (prevalence of diabetes, smoking)
- Incidence - the number of NEW cases of a condition in a defined population over a specified period of time (annual incidence of HIV infections, cancer)
- Crude and specific rates

Measurement errors

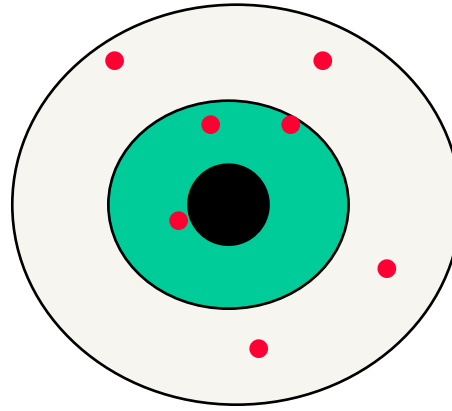
- Random error (low precision)
 - An error of measurement as a consequence of recording the value $X + \varepsilon$ instead of the true value X
- Systematic error (low validity)
 - Any systematic error that results in an incorrect estimate of the association between exposure and disease

Quality of an estimate

Precision & validity

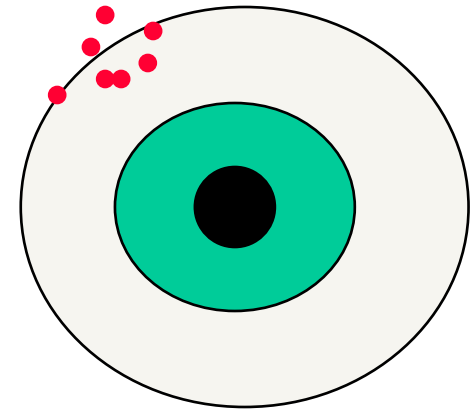


No precision



Random error

Precision but no validity

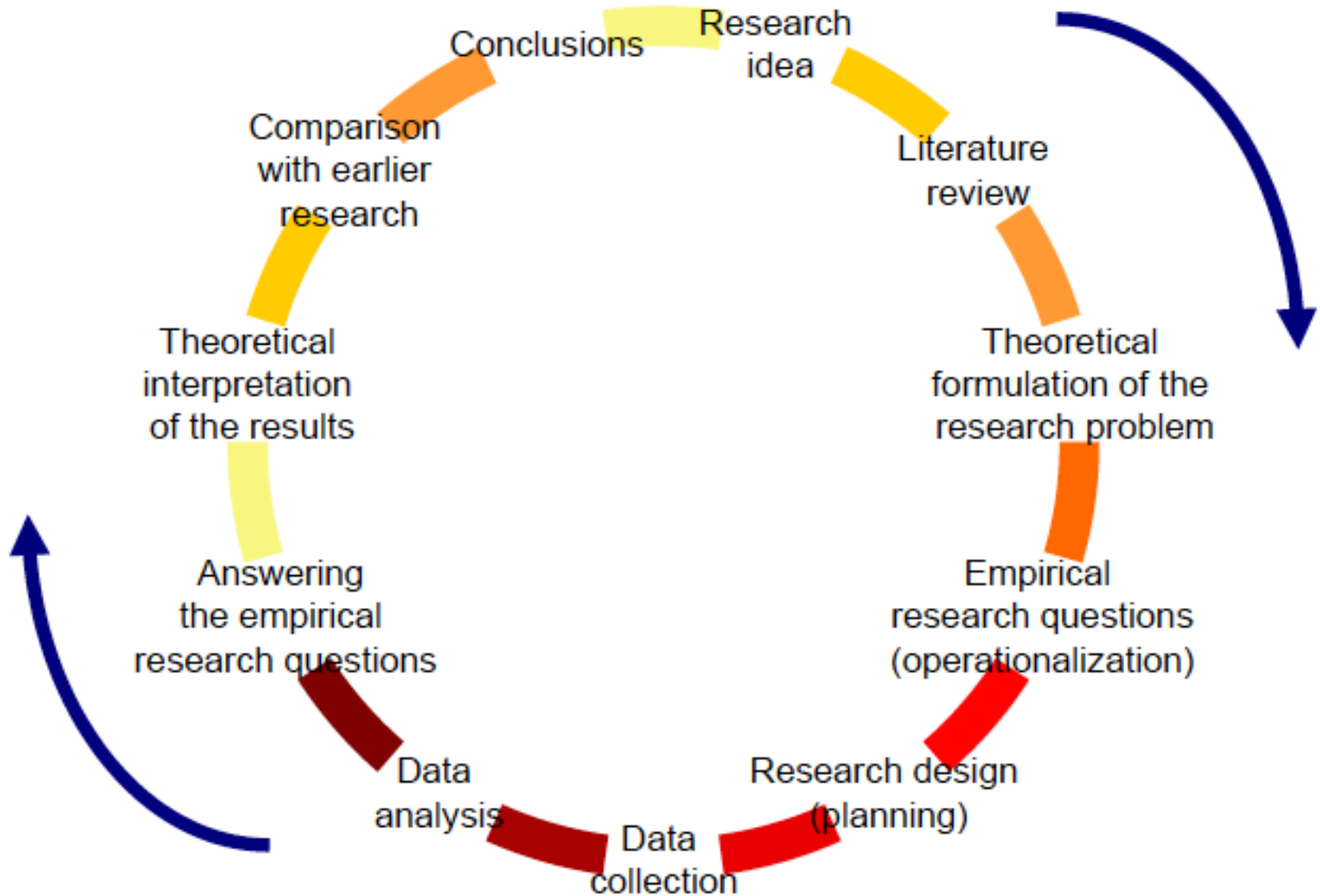


Systematic error (Bias)

Research process

- Define the problem: ask a well focused question
 - your hypothesis
- Identify the cause: test the hypothesis
 - is there an association between exposure and outcome
- Interpret the results:
 - identify/minimize threats to validity (bias, confounding),
 - role of chance

Research process



Literature review: Types of reviews

- Narrative (traditional)
- Systematic
- Meta-analysis

Differences between Narrative Reviews and Systematic Reviews*

Feature	Narrative Review	Systematic Review
Question	Often broad in scope	Focused on clinical question
Sources & Search	Not usually specified	Comprehensive sources and explicit search strategy
Selection	Not usually specified	Criterion-based selection, uniformly applied
Appraisal	Variable	Rigorous critical appraisal
Synthesis	Often qualitative	Quantitative summary**
Inferences	Sometimes evidence-based	Usually evidence-based

* Cook DJ, Mulrow CD, Haynes RB. Systematic Reviews: Synthesis of Best Evidence for Clinical Decisions. Annals of Internal Medicine. 126(5):376-80, 1997.

** Statistical synthesis (meta-analysis)

What is the study design?

- The purpose of the study design is to transform the conceptual hypothesis into an operational hypothesis that can be empirically tested
- All study designs are potentially flawed
- Important to understand the specific strengths and limitations of different study designs

Research methods

- Research methods are generally categorized as being either quantitative or qualitative.
- What matters is that the methods used fit the intended purposes of the research

Main study designs (quantitative)

- Descriptive studies
 - Examine patterns of disease
- Analytical studies
 - Studies of suspected causes of diseases
- Experimental studies
 - Compare treatment modalities

Hierarchy of Epidemiologic Study Design

Case reports

Generate hypotheses

Case series

Ecologic studies

Cross-sectional studies

Case-control studies

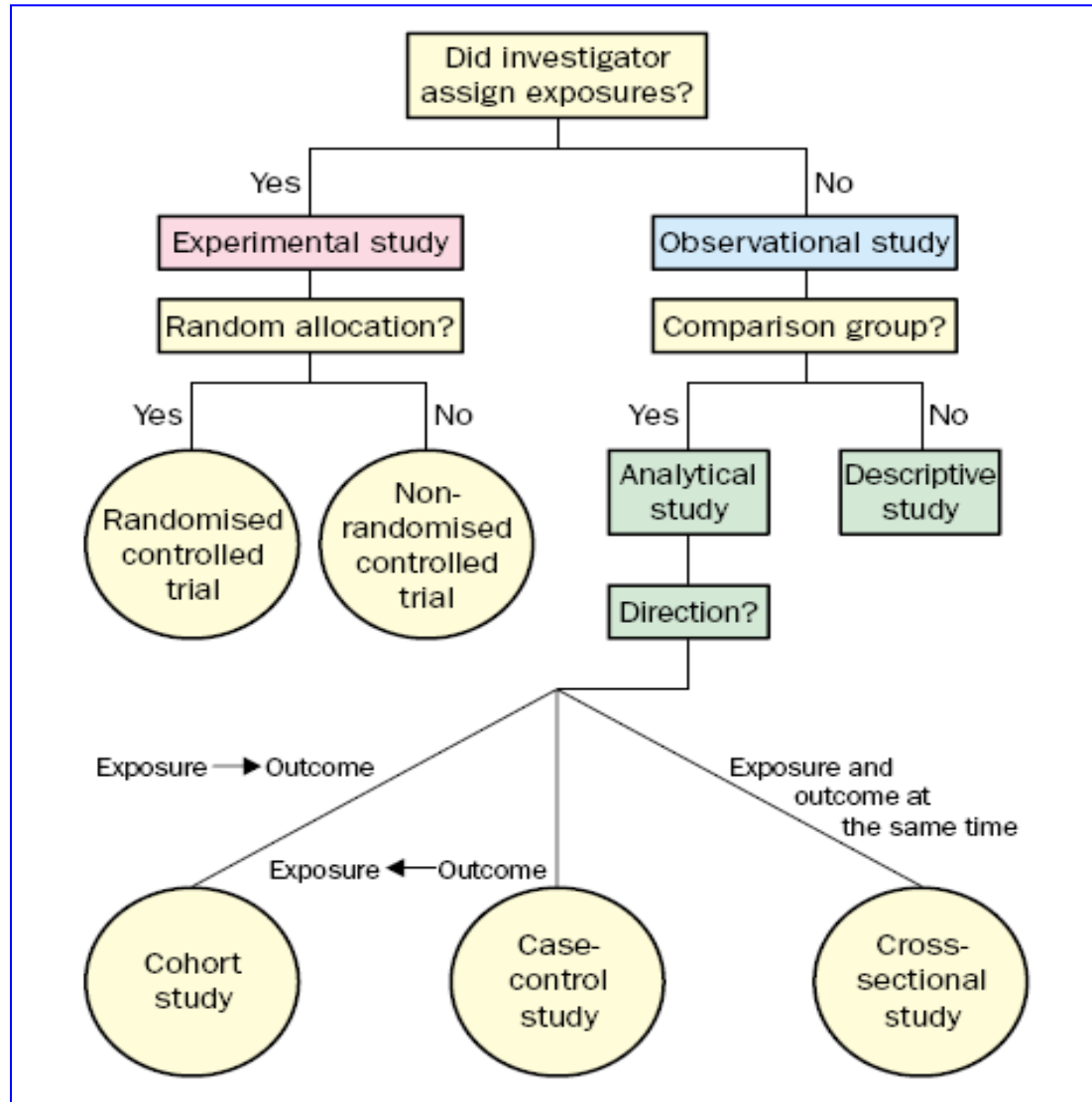
Cohort studies

Randomized controlled trials

Establish causality



Epidemiologic Study Designs



Observational studies

- Descriptive Studies look at the natural history of disease, are useful for allocation of resources or can suggest a hypothesis
 - Descriptive studies can be a case report, case series, ecologic study, or of cross-sectional design
- Analytic Studies test hypotheses and may assess causation
 - Examples of analytic study designs are case-control or cohort studies

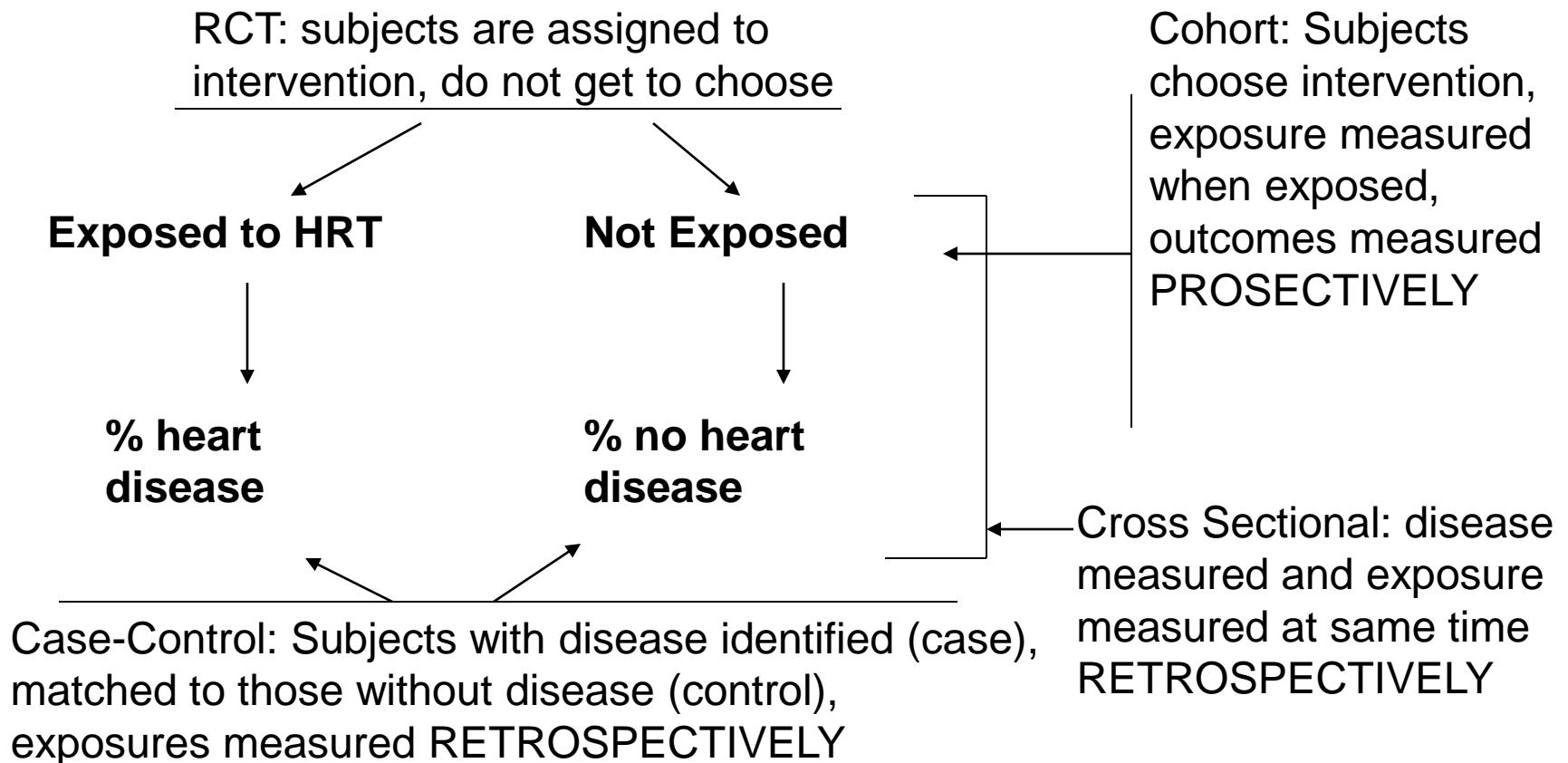
Experimental studies

- Clinical Trials test the effect (efficacy) of a treatment, such as drugs, dietary regiment of physical therapy
- Community Intervention Trials asses the effectiveness of prevention/screening programs such as a needle exchange program

Selecting study design

- Because some research questions can be answered by more than one type of research design, the choice of design depends on a variety of considerations, including:
 - availability of time
 - availability of resources
 - availability of data
- You should always aim for a design that generates the evidence to answer the initial question as unambiguously as possible

Does HRT Prevent or Cause Heart Disease?



For ALL studies ask yourself

- Are the results VALID- or is there another explanation
- VALID = TRUE

Case reports and case series

- Case reports and case series describe the experience of a single patient or a group of patients with a similar diagnosis.
- The collection of a case series rather than reliance on a single case report can mean the difference between formulating a useful hypothesis and merely documenting an interesting medical oddity

Case reports and case series

- Advantages include:
 - recognition of new diseases
 - formulation of hypotheses
- Disadvantages include:
 - based on the experience of one person, or just a few people,
 - the presence of any risk factor may be coincidental,
 - lack of an appropriate comparison group

Cross sectional studies

- A cross-sectional study is a study of a population at a single point in time
- Useful for determining the prevalence of risk factors and the frequency of prevalent cases of a disease for a defined population
- They are also useful for measuring current health status and planning for selected health services

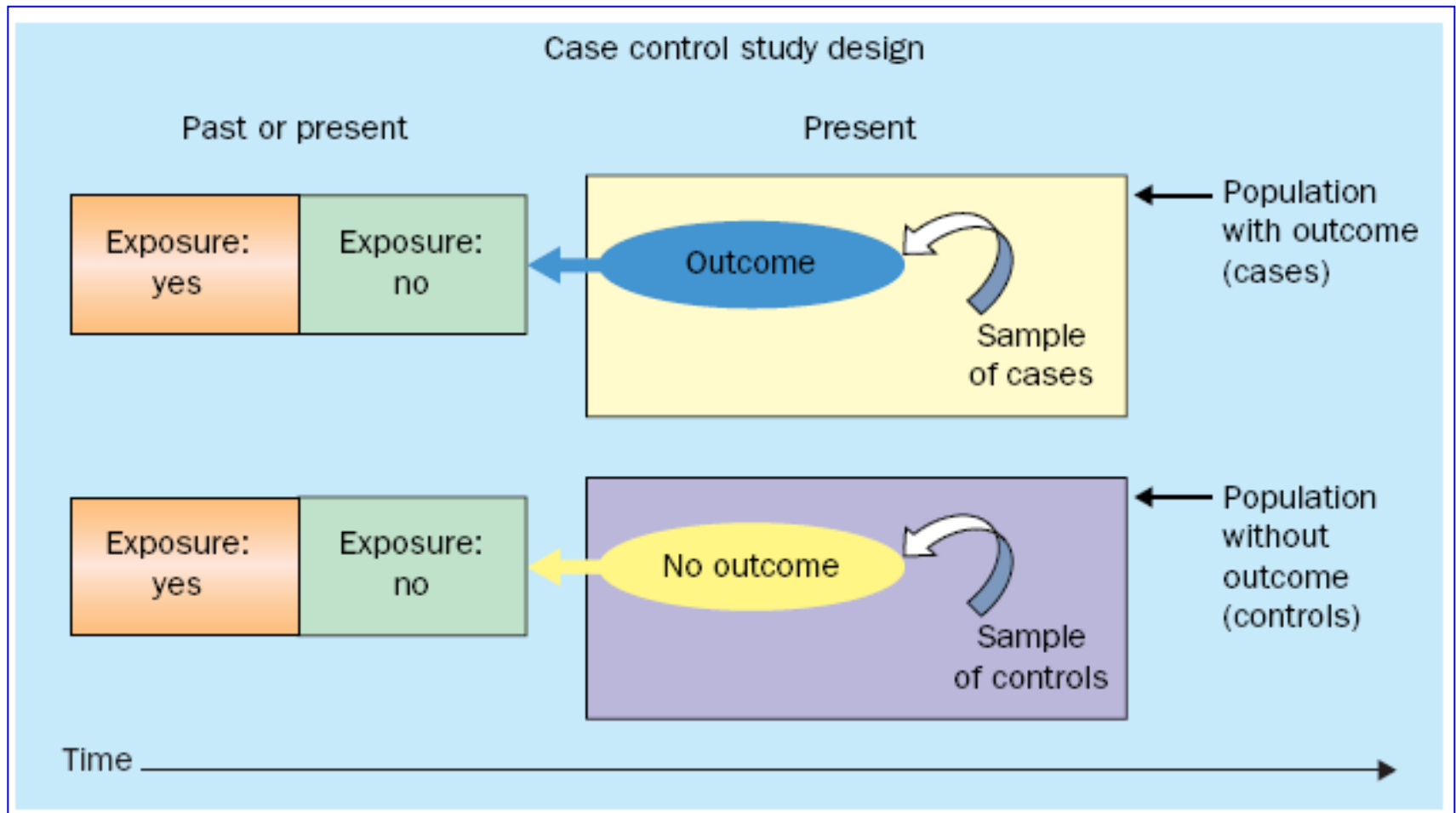
Cross sectional studies

- Advantages of a cross-sectional study include:
 - Fairly quick and easy to perform
 - Useful for hypothesis generation
- Disadvantages of a cross-sectional study include:
 - Do not offer evidence of temporal relationship between risk factors and disease
 - Not good for hypothesis testing

Case-Control studies

- The case-control study is a basic observational study
- Always retrospective
- Designed to compare risk factors in diseased and non-diseased individuals to examine possible associations

Case-Control studies



Case-Control studies

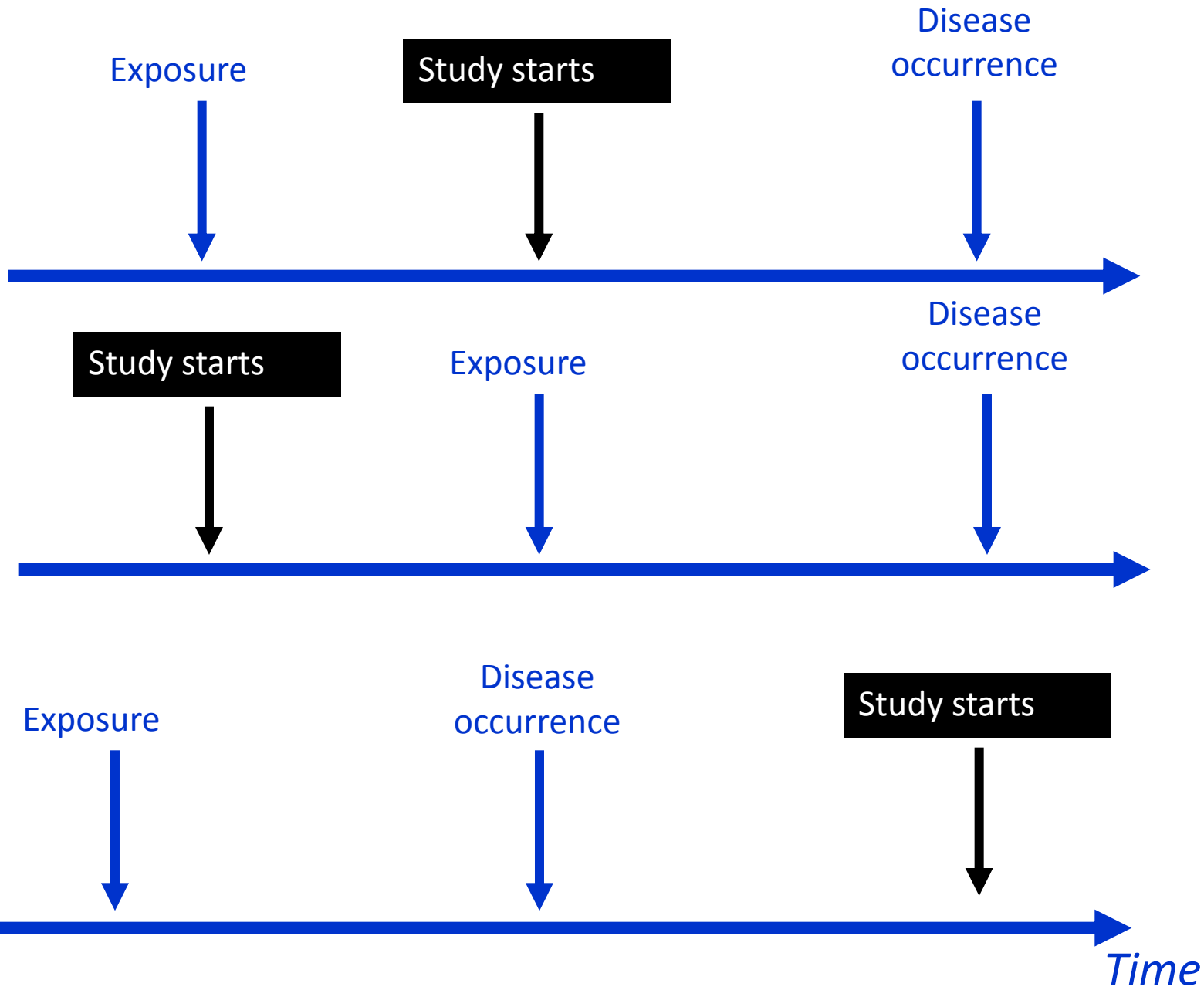
- Advantages of a case-control study include:
 - Relatively inexpensive
 - providing sufficient numbers of cases for rare diseases with long latencies
 - allowing several exposures to be evaluated at the same time
- Disadvantages of a case-control study include:
 - susceptible to selection and information bias
 - not appropriate for prevalence/incidence estimates
 - not allowing estimation of risk
 - not considering more than one disease
 - not feasible for rare exposures
 - temporal relationship between exposure and disease can be difficult/impossible to establish

Case-Control studies

- Selection of a comparison group, i.e., the controls, is an important issue when conducting a case-control study
- The ideal control group should be representative of the population from which the cases are derived, typically called the source population

Cohort studies

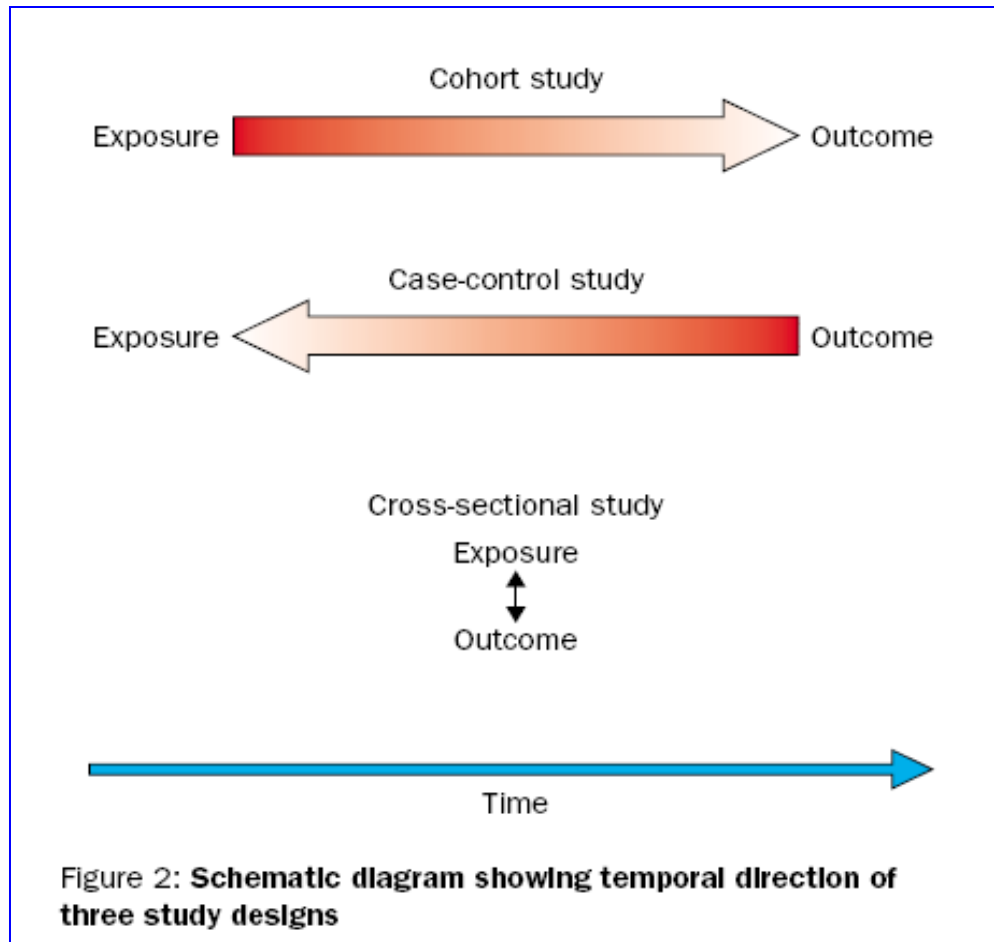
- The cohort study is a basic observational study design most similar to a clinical trial.
- Characteristics include:
 - Always a follow-up study with forward directionality
 - Can be prospective or retrospective



Cohort studies

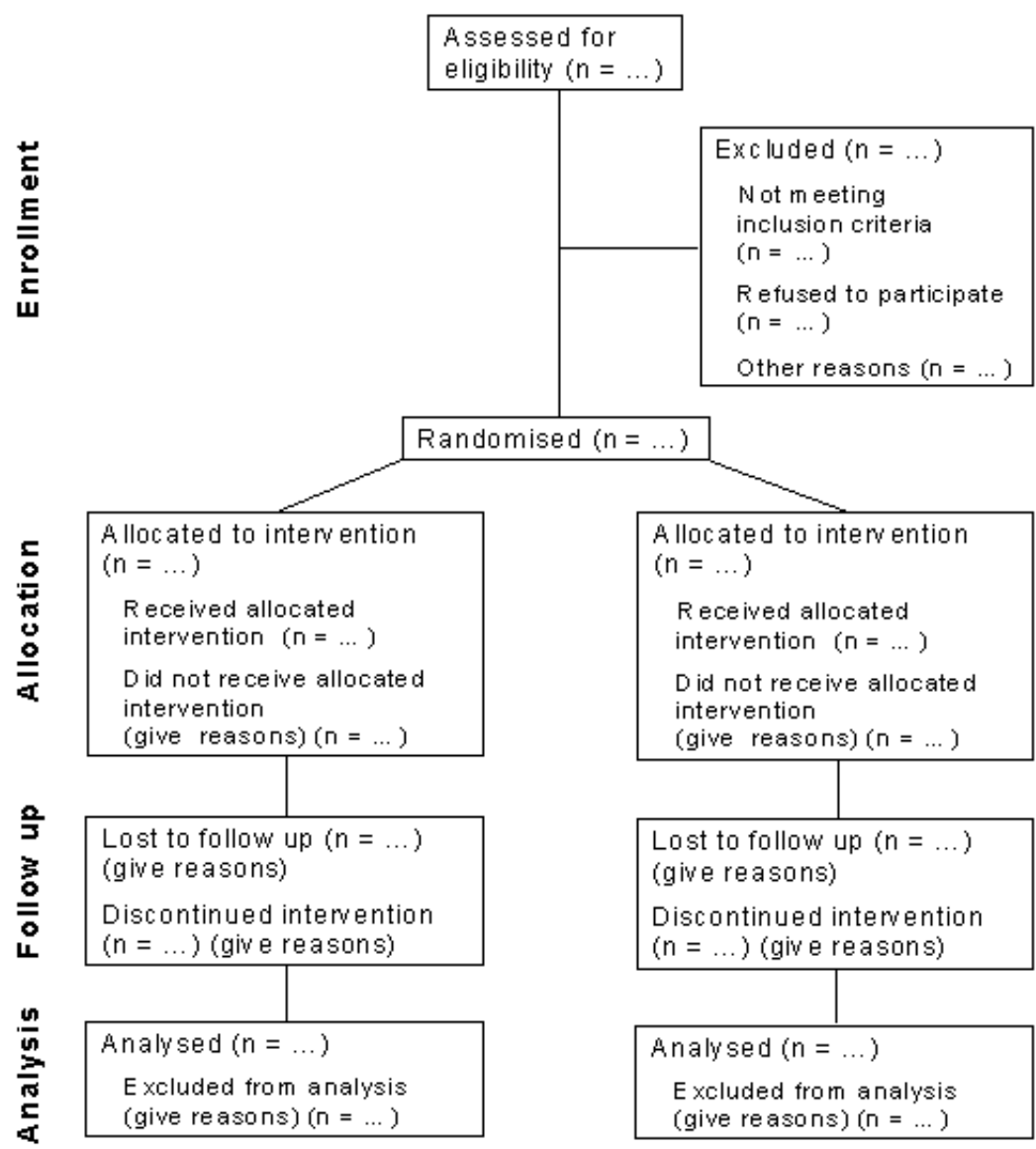
- Advantages of a cohort study include:
 - Least prone to bias when compared with other observational study designs
 - Forward directionality looks at cause before effect
 - Can study several diseases
- Disadvantages of a cohort study include:
 - Often quite costly and time-consuming, particularly if prospective
 - Loss-to-follow-up may lead to bias
 - Poor design for studying rare diseases or diseases with long latencies

Epidemiologic Study Designs



Clinical trials

- The clinical trial is the design that most closely resembles a laboratory experiment. The major objective is to test the efficacy of a therapeutic or preventive intervention.
- Key features of a clinical trial are:
 - Randomization
 - Blinding
 - Stopping rules
 - Intention to treat analysis



Clinical trials

- Key advantages:
 - “Gold standard” for evaluating treatment interventions
 - Allow the investigator to have extensive control over the research process
- Key disadvantages
 - Expensive
 - Not suitable for every question
 - May be limited in generalizability (tension between internal and external validity)

Bias

- Bias results from systematic flaws in study design, data collection, or the analysis or interpretation of results
- Are the results believable? (internal validity)
- Can results from study participants be extrapolated to the broader population? (external validity)

Types of bias

- Selection bias [Unrepresentative nature of sample]
- Information (misclassification) bias [Errors in measurement of exposure of disease]
- Confounding bias [Distortion of exposure - disease relation by some other factor]
- Types of bias are not mutually exclusive

Selection bias

- Selective differences between comparison groups that impacts on relationship between exposure and outcome
- Usually results from comparative groups not coming from the same study base and not being representative of the populations they come from

Information/measurement/misclassification bias

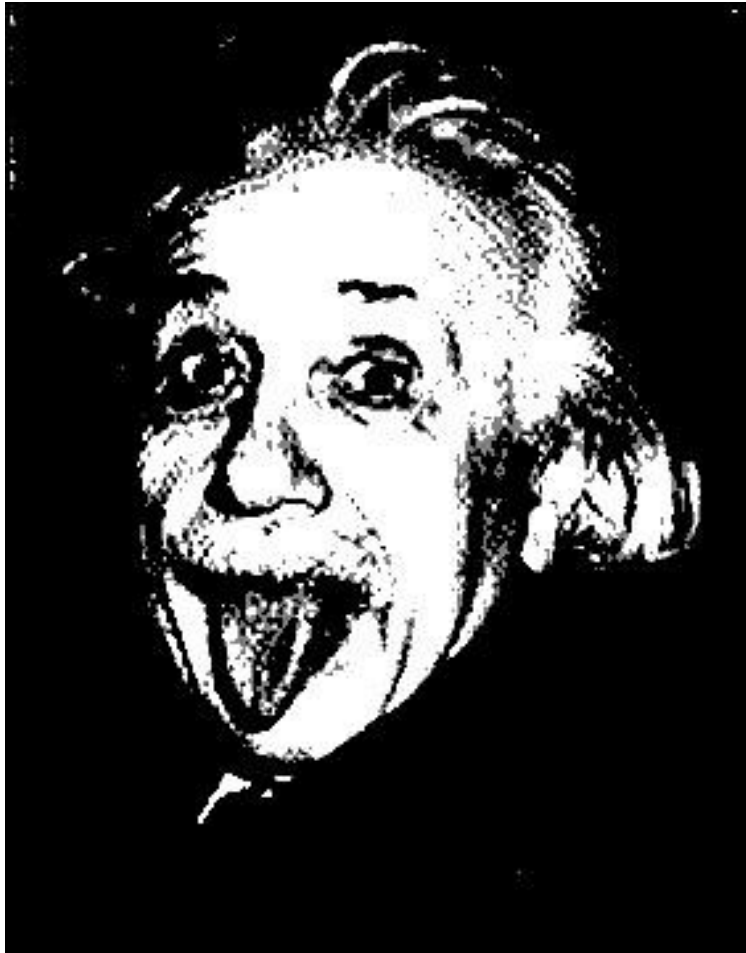
- Method of gathering information is inappropriate and yields systematic errors in measurement of exposures or outcomes

Information/measurement/misclassification bias

- Sources of information bias:
 - Subject variation
 - Observer variation
 - Deficiency of tools
 - Technical errors in measurement

Confounding

- Confounding results when the effect of an exposure on the disease (or outcome) is distorted because of the association of exposure with other factor(s) that influence the outcome under study



Not everything that
counts can be
counted, and not
everything that
can be counted
counts

Questions?