Study Designs in Epidemiology

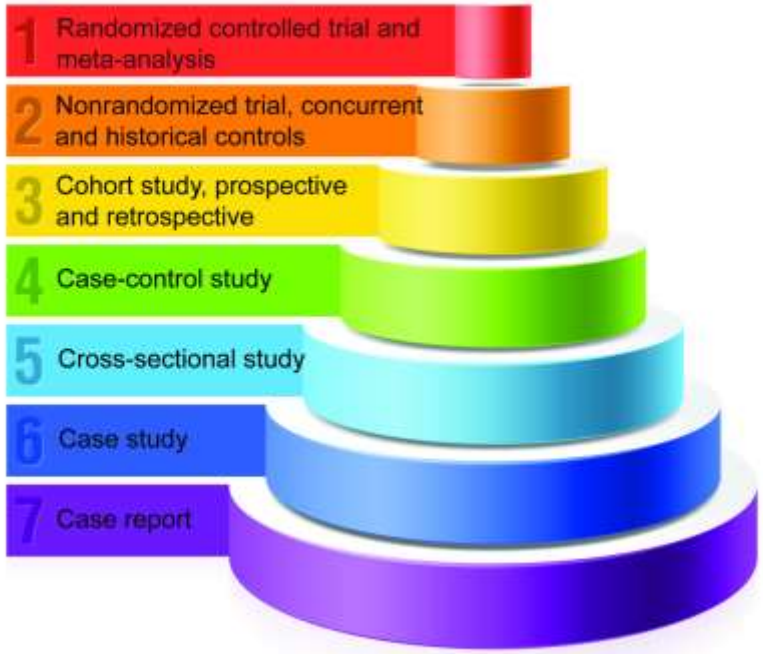

Figure. Hierarchy of Research Design

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Epidemiologic Study Design

Study design is the arrangement of conditions for the collection and analysis of data to provide the most accurate answer to a question in the most economical way.

Types of Epidemiologic Study Designs

I. Based on objective/focus/research question:

1. Descriptive studies

Describe: what, who, when, where

2. Analytic studies

Analyze: How and why

Types of Epidemiological Study Designs

II. Based on the role of the investigator

1. Observational studies

- The investigator observes what naturally happens
- No intervention

2. Intervention/Experimental studies

- Investigator intervenes: changes things and introduce exposure.
- Researcher has control over the situation

Types of Epidemiological Study Designs

III. Based on timing :

1. One-time (one-spot) studies

- Conducted at a point in time
- An individual is observed at once

2. Longitudinal (Follow-up) studies

- Conducted over a period of time
- Individuals are followed over a period of time

Types of Epidemiological Study Designs

IV. Based on direction of follow-up/data collection:

1. Prospective

Data collection occurs forward in time: into the future

2. Retrospective

Conducted backward in time: past events

Types of Epidemiological Study Designs

V. Based on type of data they generate:

1. Qualitative studies:

- Generate textual data
- Also called exploratory studies

2. Quantitative studies:

- Generate numerical data
- Also called explanatory studies

Types of Epidemiological Study Designs

The most widely used classification:

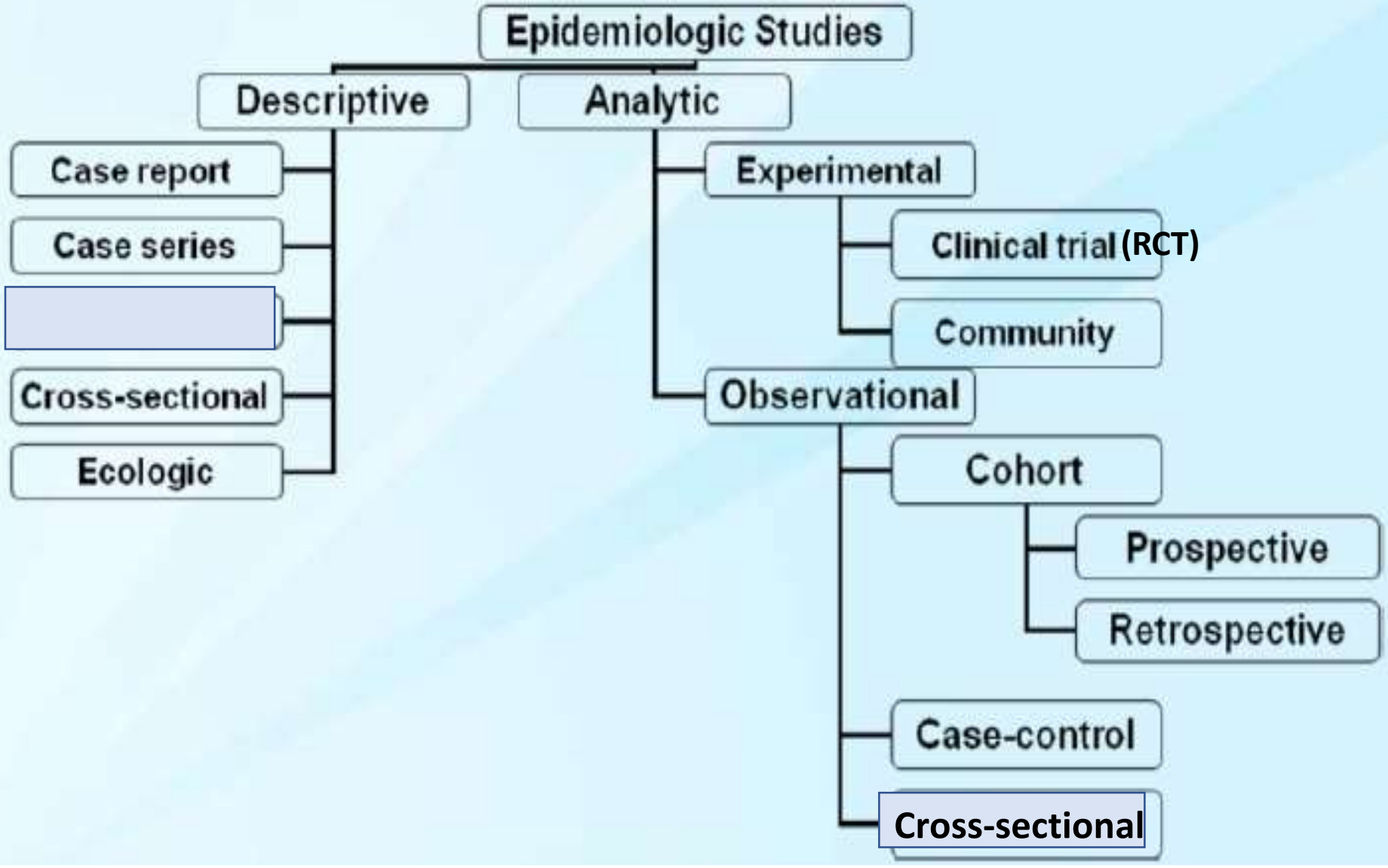
- **Descriptive studies (who, when, where)**
  describe occurrence of outcome

- **Analytic studies (how, why)**
  describe **association** between exposure and outcome

Basic Research Study Designs in Epidemiology

Study design is the arrangement of conditions for the collection and analysis of data to provide the most accurate answer to a question in the most economical way.

Taxonomy of Epidemiologic Studies



Descriptive Studies

- Descriptive studies are usually the first phase of an epidemiological investigation.
- These studies are concerned with observing the distribution of disease or health – related characteristics in human populations.
- Such studies basically ask the questions of what, who, where, and when.
- Useful for generating new hypothesis (provides clues to disease etiology)

Research Hypothesis

A hypothesis is a supposition, arrived at from observation or reflection.

- It can be accepted or rejected using the techniques of analytical epidemiology.

A hypothesis should specify the following:

1. The population.
2. The specific cause being considered.
3. Expected outcome – disease.
4. Time response relationship (expectation).
5. Be understandable, measurable and testable.

Develop a research question & Hypothesis

- General concern – Hb of mother and Birth weight of baby.

RQ -

- Is Anemia in pregnancy associated with low birth weight in newborn?

Null Hypothesis

- There is no difference in the incidence of LBWs in the mothers who are anemic and those who are not anemic.

Research Hypothesis

- The incidence of LBWs in mothers who are anemic is higher than those who are not anemic

Descriptive studies

1. Case Reports:

- presentation of a single case or handful of cases
- Generally report a new or unique finding
    - e.g. previous undescribed disease
    - e.g. unexpected link between diseases
    - e.g. unexpected new therapeutic effect
    - e.g. adverse events

Descriptive studies

2. Case Series

Experience of a group of patients with a similar diagnosis

- Cases may be identified from a single or multiple sources
- Generally report on new/unique condition
- May be the only realistic design for rare disorders

Case report and Case Series

- Advantages
  - Useful for hypothesis generation
  - Informative for very rare diseases with few established risk factors
- Disadvantages
  - Cannot study cause and effect relationships
  - Cannot assess disease frequency in a population

3. Ecological Studies (correlation study)

The ecologic study is a hypothesis generating study. Usually using group-level data (population-level), it examines if two factors are correlated with each other.

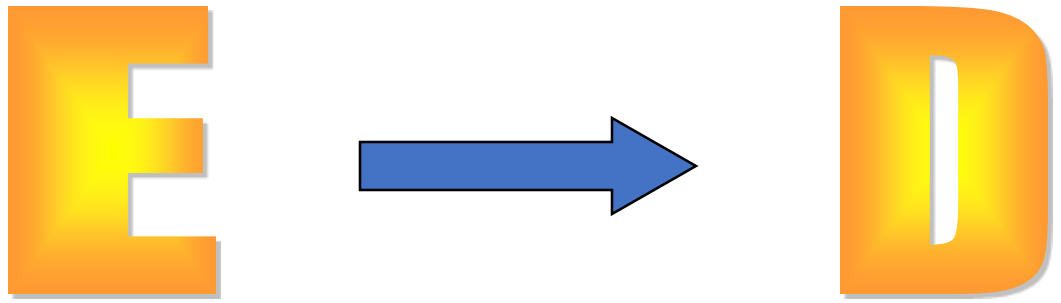
- It involves the collection of events over a defined population base and by the use of denominator data to determine rates.

It results in Ecological Fallacy: Failure in reasoning that arises when an inference is made about an individual based on aggregate data for a group

e.g. Higher rates of coronary heart disease in countries with higher income, Higher rates of leukemia in larger cities , higher rates of car accidents in countries or regions with higher smoking rates.

Analytical Epidemiology

Are exposure and disease linked?



Exposure **Disease**

Analytical Studies (testing hypothesis)

Observational Studies

- Cross-sectional
- Case-control
- Cohort

Experimental Studies

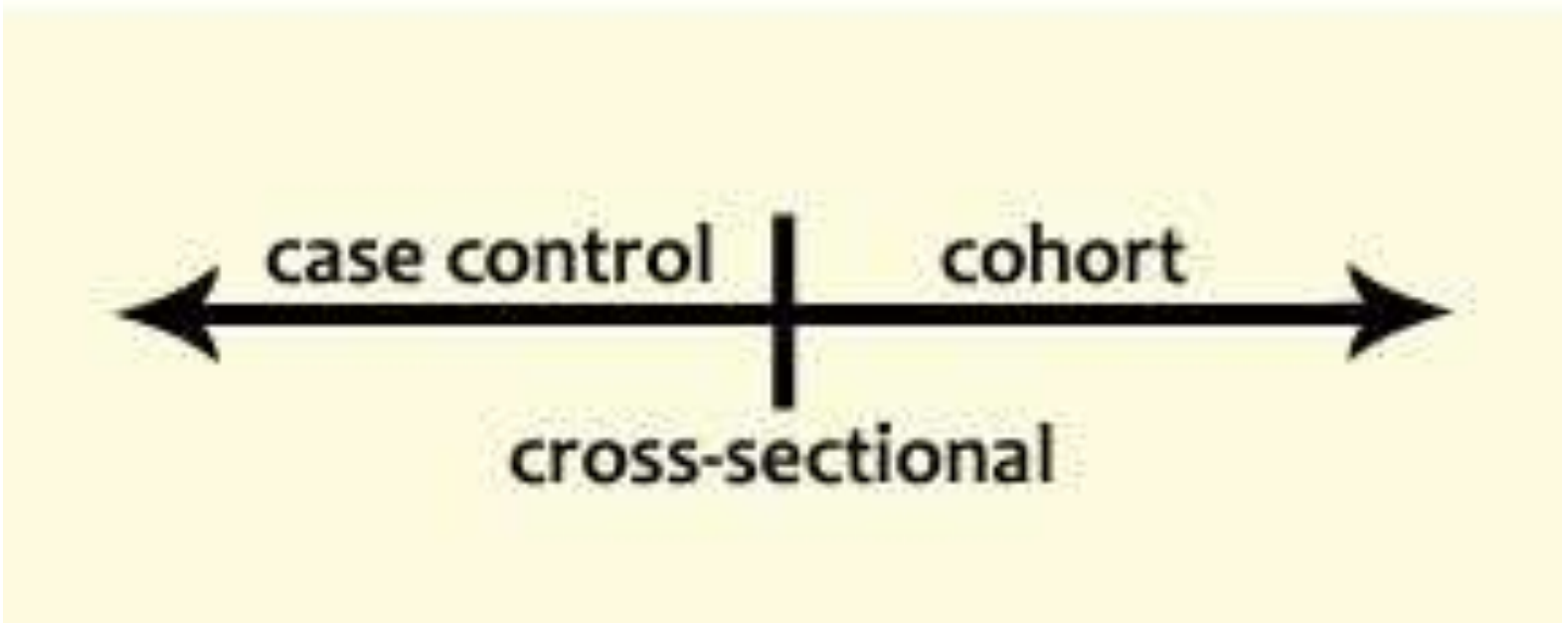
- Randomized controlled clinical trials
- Community trials

Observational Studies

Non-experimental study designs:

- Observational because there is no individual intervention
- Treatment and exposures occur naturally
- Individuals can be observed prospectively, retrospectively, or currently

Observational Studies

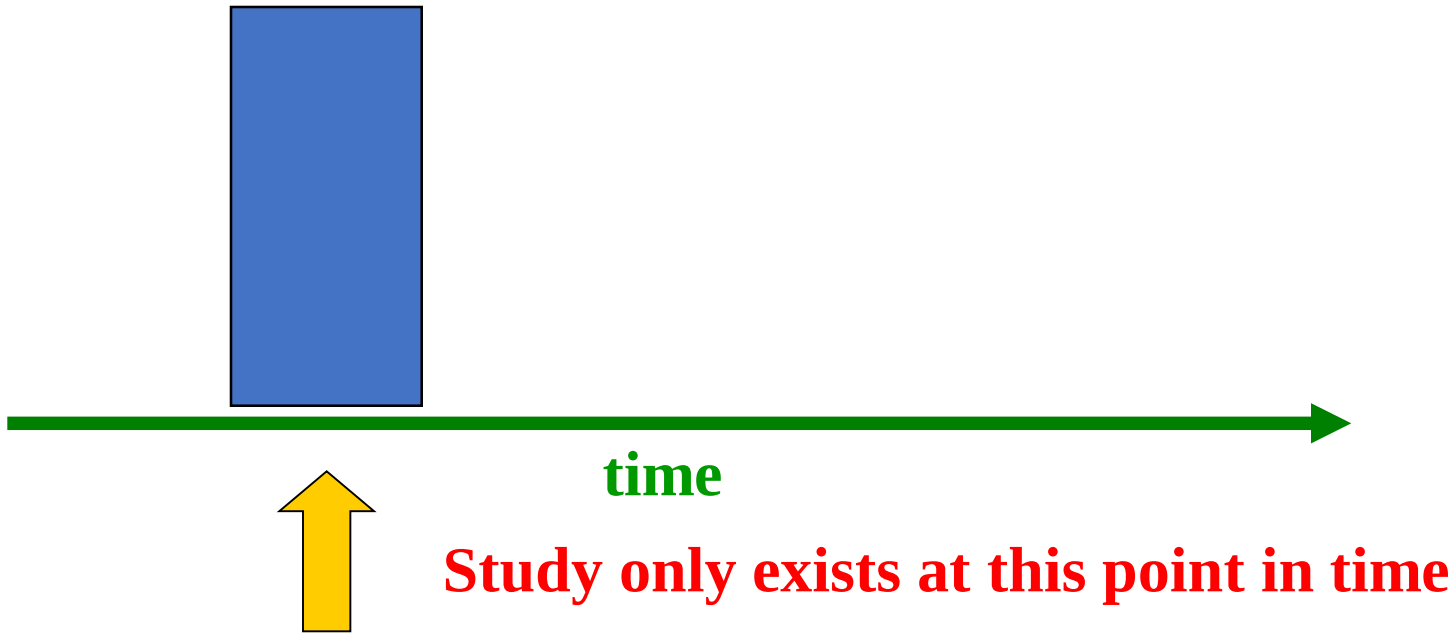


http://www.medbullets.com/step1-stats/1001/types-of-studies

Cross-sectional studies

An “observational” design that surveys exposures and disease status at a single point in time (a cross-section of the population)

It is named “prevalence study”



Cross-sectional studies

- Based on a single examination of a cross section of population at one point in time, by studying a sample that represents the population.
- Results of CS study can be generalized to the whole population (provided the sampling has been done correctly).
- Longitudinal studies are Based on multiple observations in the same population over a multiple points of time.

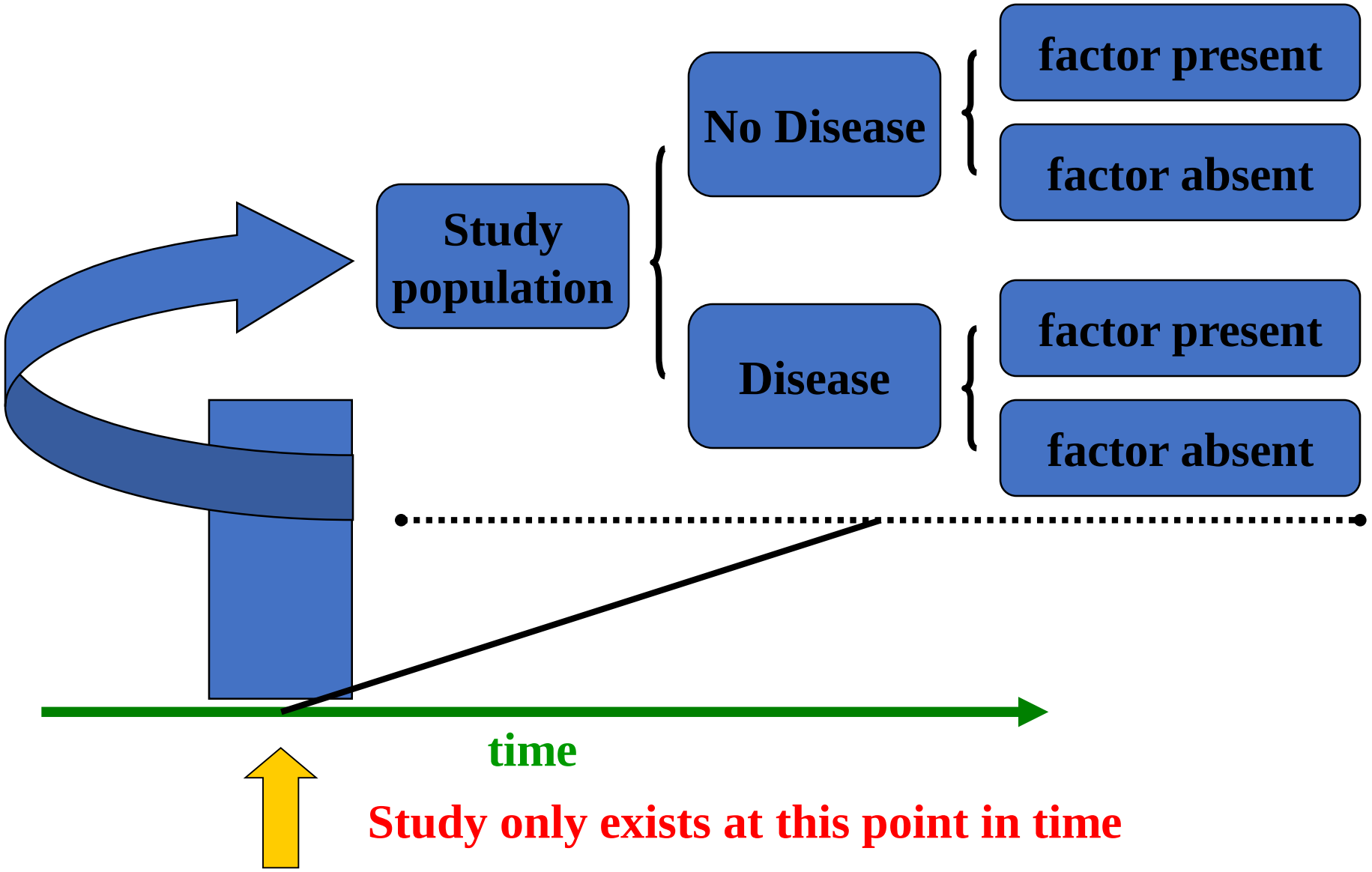
e.g. What is the prevalence of diabetes in Jordan?
What is the prevalence of malnutrition among children in Jordan?
A survey of asthma among animal handlers
A survey of dietary habits among university students

Cross-sectional studies

Used to learn more about the disease to explore factors that have role in the etiology of the disease:

- Physical characteristics of people, material and environment
- Socio-economic characteristics e.g., age, education , marital status, number of children and income
- Behavior or practices of people, knowledge, and attitude and beliefs (KAP)
- Events that occur in population

Cross-sectional Design



Cross-sectional Studies

- Are the simplest form of observational studies.
- Often used to study conditions that are relatively frequent with long duration of expression (nonfatal, chronic conditions)
- It measures prevalence, not incidence of disease
- Example: community surveys
- Not suitable for studying rare or highly fatal diseases or a disease with short duration of expression.

Cross-sectional…

Advantages of cross-sectional studies

- Less time consuming
- Less expensive
- Provides more information (lots of variables)
- Describes the population well
- Generates hypothesis

Cross-sectional study provides a snap-shot or a photograph of a population at a certain point in time.

Cross-sectional studies

Disadvantages

- Weakest observational design, (it measures prevalence, not incidence of disease). Prevalent cases are only the survivors.
- The temporal sequence of exposure and effect may be difficult or impossible to determine.
- Usually don't know when disease occurred
- Rare events a problem.
- Quickly emerging diseases are also a problem.
- Least useful in establishing causation (among analytical studies).

Is Cross-sectional design Descriptive or Analytical?

- It may be difficult to decide whether the disease or the exposure came first, so causation should always be confirmed by stronger studies.
- The collection of information about risk factors is retrospective, running the risk of recall bias.
- In practice cross-sectional studies include elements of both descriptive and analytical design.

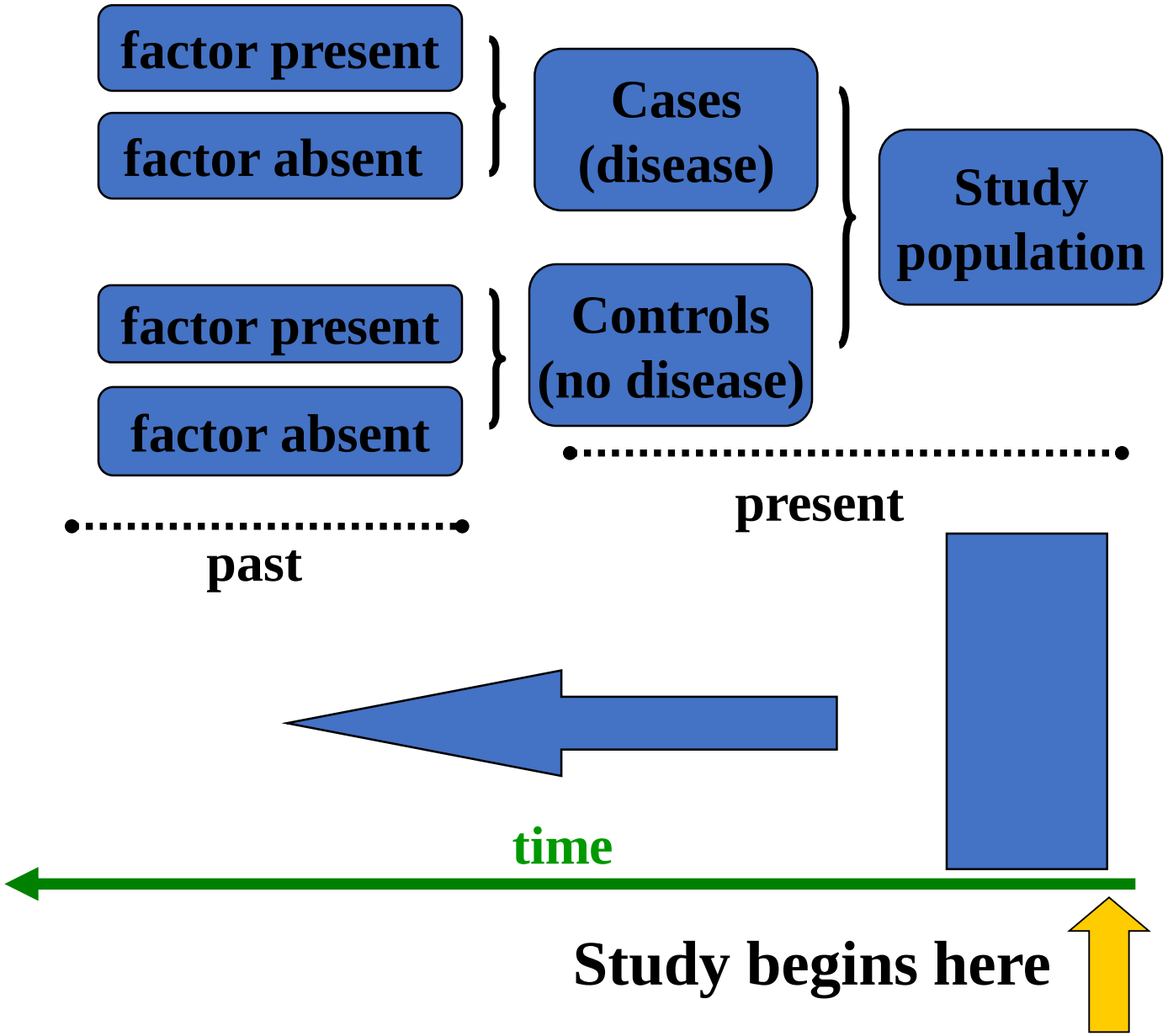
Case-Control Study Design

The investigator compares one group among whom a health problem is present with another group, called a control or comparison group, where the health problem is absent to find out what factors have contributed to the problem.

e.g. A study to explore the relationship between obesity and breast cancer.

e.g. A study to assess the effect of mothers' educational level on malnutrition among children

Case-Control Design



Case-Control Studies

An “observational” design comparing exposures in disease cases vs. healthy controls from the same population.

- exposure data collected retrospectively.
- most feasible design where disease is rare.
- This is the first approach to test causal hypothesis.
- Definition of a case is crucial to a case control study.

SELECTION OF CONTROLS

- The controls must be free from the disease under study.
- They must be similar to the cases as possible, except for the absence of the disease under study (matching).
- Each case needs one control or more.

Selection of an appropriate control group is an important pre requisite, because we will be making comparison with these controls..

Case-Control Study

Strengths:

1) Less expensive and less time consuming
2) Efficient for studying rare diseases
3) Allows the study of several different etiological factors for one disease.
4) No attrition problems (no follow-up).
5) Ethical problems are minimal (no risk to participants)

Case-Control Study

Limitations

1. Selection of an appropriate control group may be difficult.
2. Inefficient for evaluation of rare exposure
3. Difficult to establish temporal sequence
4. Determining exposure will often rely on memory, leading to bias (recall bias).
5. We cannot measure incidence & can only estimate the relative risk (RR).

Cohort Study

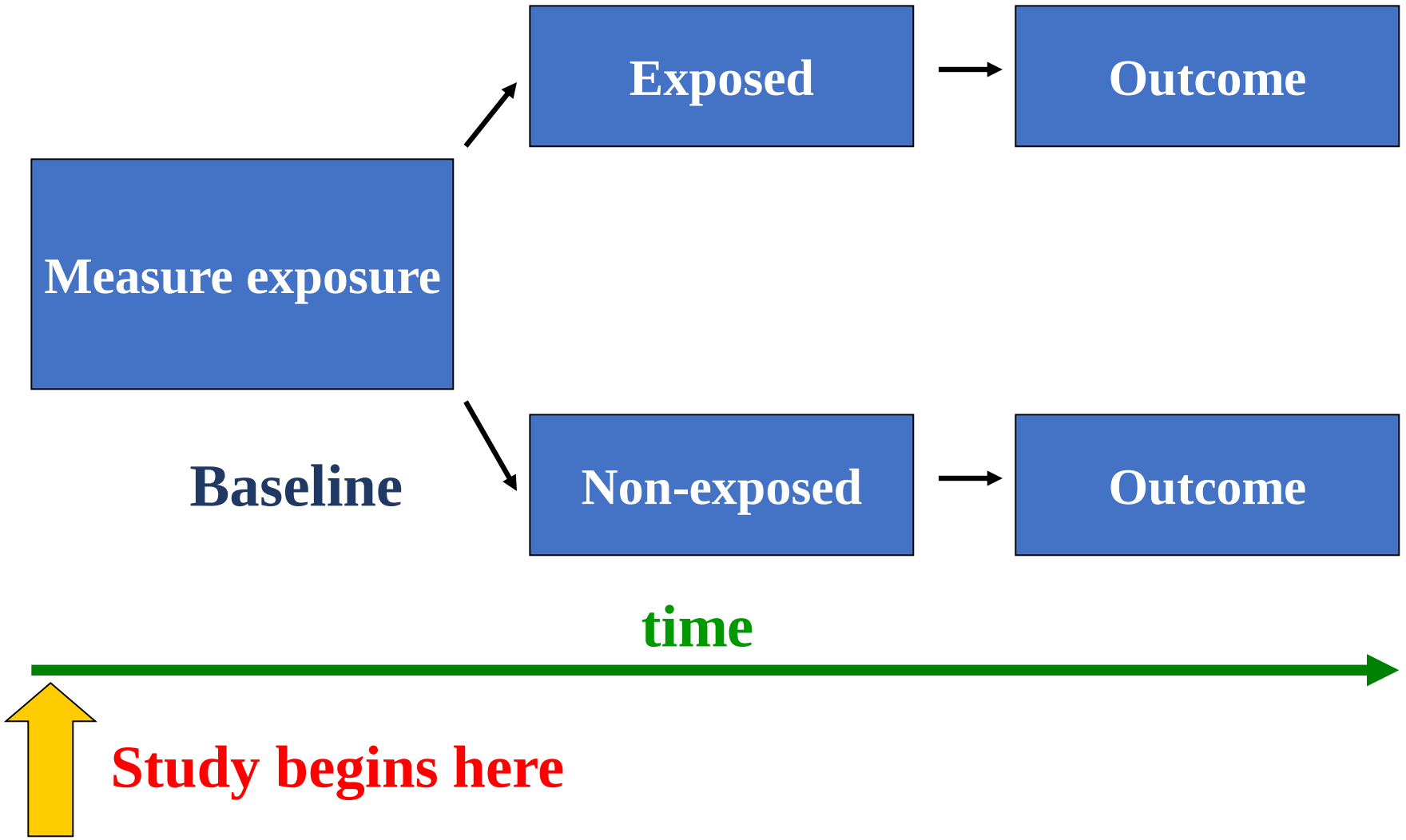
In a COHORT STUDY, a group of individuals that is exposed to a risk factor (study group) is compared with a group of individuals not exposed to the risk factor (control group)....and all followed up to monitor occurrence of disease.

- Cohort study is known by a variety of names: prospective study, longitudinal study, incidence study & forward looking study.

  e.g. Does living in poor housing increases the risk of developing cancer?

  Does following a healthy life style lower the risk of hypertension?.

Prospective Cohort study



Cohort Study

Is an “observational” design comparing individuals with a known risk factor or exposure with others without the risk factor or exposure.

- Looking for a difference in the risk (incidence) of a disease over time.
- Best (strongest) observational design.
- Data usually collected prospectively (some retrospective).

Cohort Study

Indications:

- When there is a good evidence of an association between exposure & disease.
- When exposure is rare, but incidence is high among the exposed.
- When attrition of the study population can be minimized (due to long follow-up period).
- When ample funds are available (it is expensive).

Advantages of cohort studies

1. Valuable when exposure is rare
2. Examines multiple outcomes of a single exposures
3. Temporal relationship is known
4. Allow direct measurement of risk
5. Minimize bias in ascertainment of exposure
   - ✓ Exposure status determined before disease detection (avoid information bias).
   - ✓ Subjects selected before disease detection (avoid selection bias).

Limitations of Cohort Study

1. Expensive
2. Time-consuming
3. Inefficient for rare diseases or diseases with long latency
4. Loss to follow-up is a problem

Framingham Study

What is the Framingham study?

When did it start? Where?

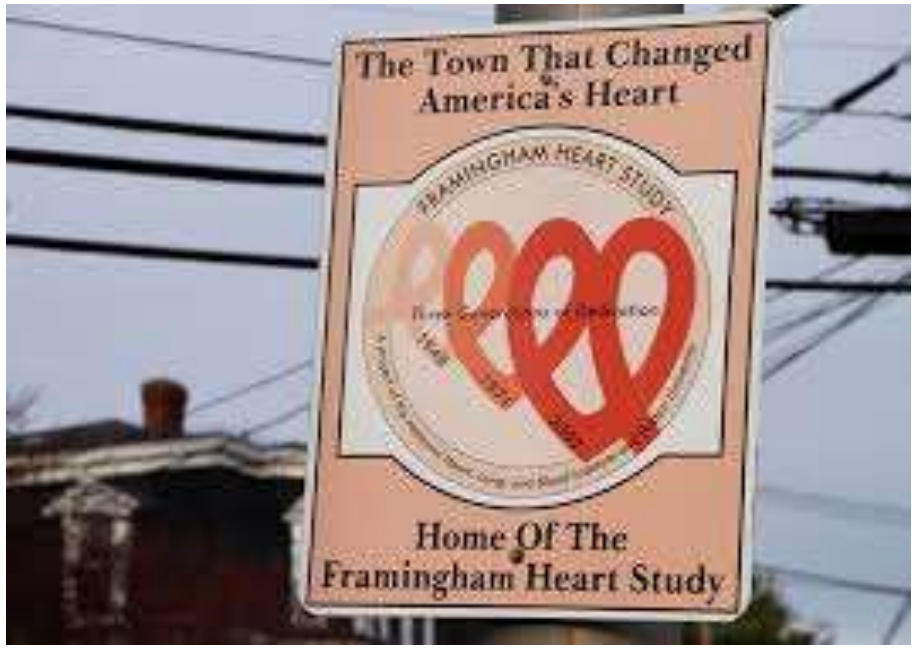
What was the disease studied?

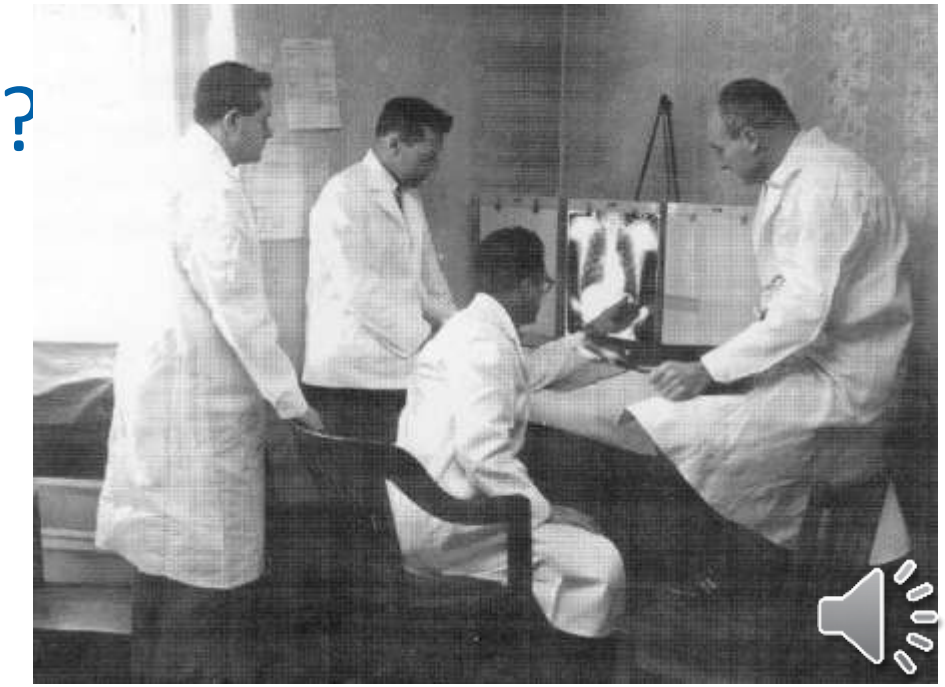
What are the most important findings?

How many people participated?

How many generations?

When did it end?





Experimental Studies (Intervention studies)

- In an experiment, we are interested in the effect or consequences of a new therapeutic treatment or procedure on an outcome.
- The subjects are allocated into a treatment group and a control group (old treatment or placebo).

Intervention: The researcher administers the exposure (treatment) to the subjects

Types of experimental studies:

1. **Randomized Controlled Trial**: on patients in clinical settings **(e.g. RCT).**
2. **Quasi-experimental:** Natural experiments, Field trial, Community trial (new Covid-19 vaccine), cross-over studies.

RCT (Randomized Controlled Trial)

Randomized Controlled Clinical trials are the most well known experimental design.

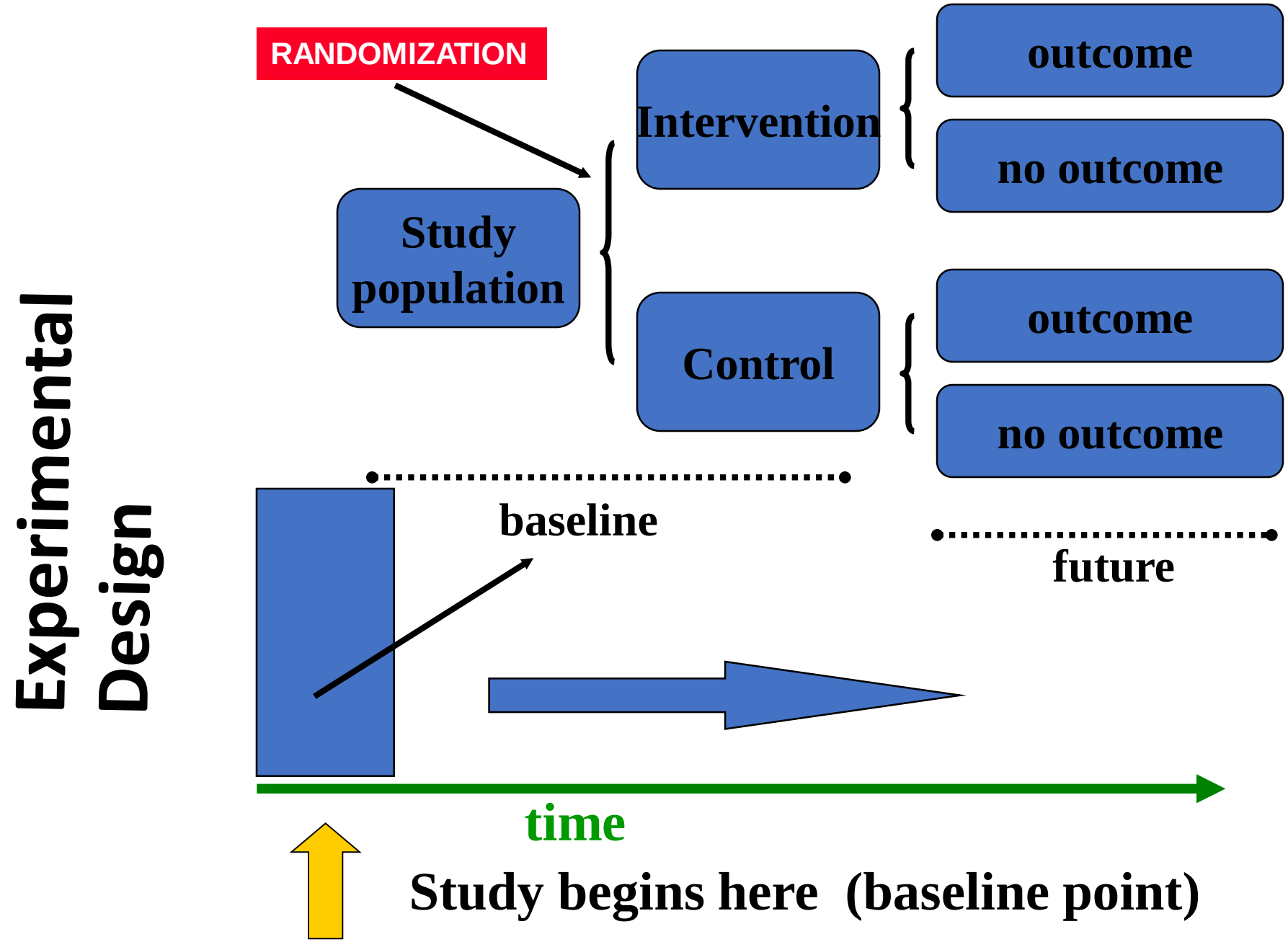
RCT is a clinical trial that is well-designed (controlled and randomized).

Controlled means: The researcher manipulates situations/objects.

An experimental design with subjects randomly assigned by the investigator into a “treatment” group and a “comparison” group.

The ultimate form of design in **testing causal hypotheses** (provides most convincing evidence).

Experimental Design



Randomised Controlled Clinical Trials (RCT):

If properly done, experimental studies can produce high quality data. Thy are the **gold standard** study design (strongest, most robust).

The quality of this “Gold standard” in experimental studies can be achieved through:

Randomization, Blinding, and use of Placebo.

e.g. The effectiveness of a new treatment for rheumatoid arthritis.
e.g. Comparing the length of stay in hospital between laparoscopy and surgery for appendicitis.

Randomization: random allocation of study subjects in to treatment & control groups. Avoids bias & confounding, and increases confidence in the results.

Blinding: Denying information on treatment / control status (single, double or triple blinding). This helps to avoid reporting bias, observation bias and assessment bias.

Placebo is used as blinding procedure …..an pharmacuologically inert material indistinguishable from active treatment. Used to avoid Placebo effect: tendency to report favourable response regardless of physiological efficacy.

Randomized Controlled Trials

Disadvantages of RCTs:

- Very expensive
- Not appropriate to answer certain types of questions for ethical reasons:

  It may be unethical, for example, to assign persons to certain treatment or comparison groups if exposure has well-known benefit.

Randomized Controlled Trials (RCTs)

It is not unexpected to find that observational studies find different results than for clinical trials.

Clinical Trials of hormone replacement therapy in menopausal women found no protection for heart disease, contradicting findings of 100's of prior observational studies.

Example of experimental design

It can be used to evaluate preventive strategies experimentally.

- Factories participating in a coronary heart disease prevention project were assigned to two groups, one receiving a programme of screening for coronary risk factors and health education, and the other being left alone.
- Subsequent disease incidence was then compared between the two groups.
- The main application of experimental studies, however, is in evaluating therapeutic interventions by randomised controlled trials.

Example of experimental design

- In a trial to prevent onset of diabetes among high-risk individuals, investigators randomly assigned enrollees to one of three groups — placebo, an anti-diabetes drug, or lifestyle intervention.
- At the end of the follow-up period, investigators found the lowest incidence of diabetes in the lifestyle intervention group, the next lowest in the anti-diabetic drug group, and the highest in the placebo group.

Quasi-Experimental Studies

The researcher does not decide or plan the intervention (e.g. changes in using health care after removing ophthalmic services from health insurance), no Randomization or no control group.

Natural experiments

Factor occured naturally : e.g. Increase in mental disorders following an earthquack.

Crossover Studies participant work as a control for himself (e.g. New pain reliefmedication)