Study Designs in Epidemiology

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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 nature
- No intervention

2. Intervention/Experimental studies

- Investigator intervenes: changes things
- He has a 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

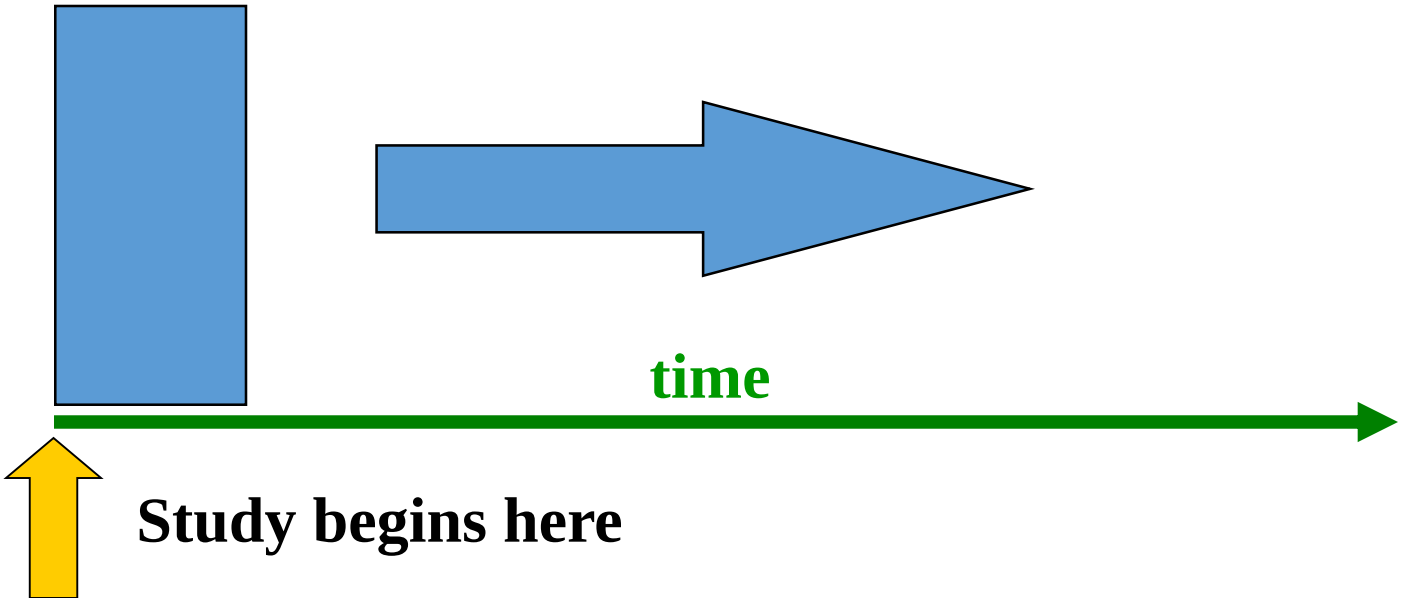
   Conducted forward in time: into the future

2. Retrospective

   Conducted backward in time: past events

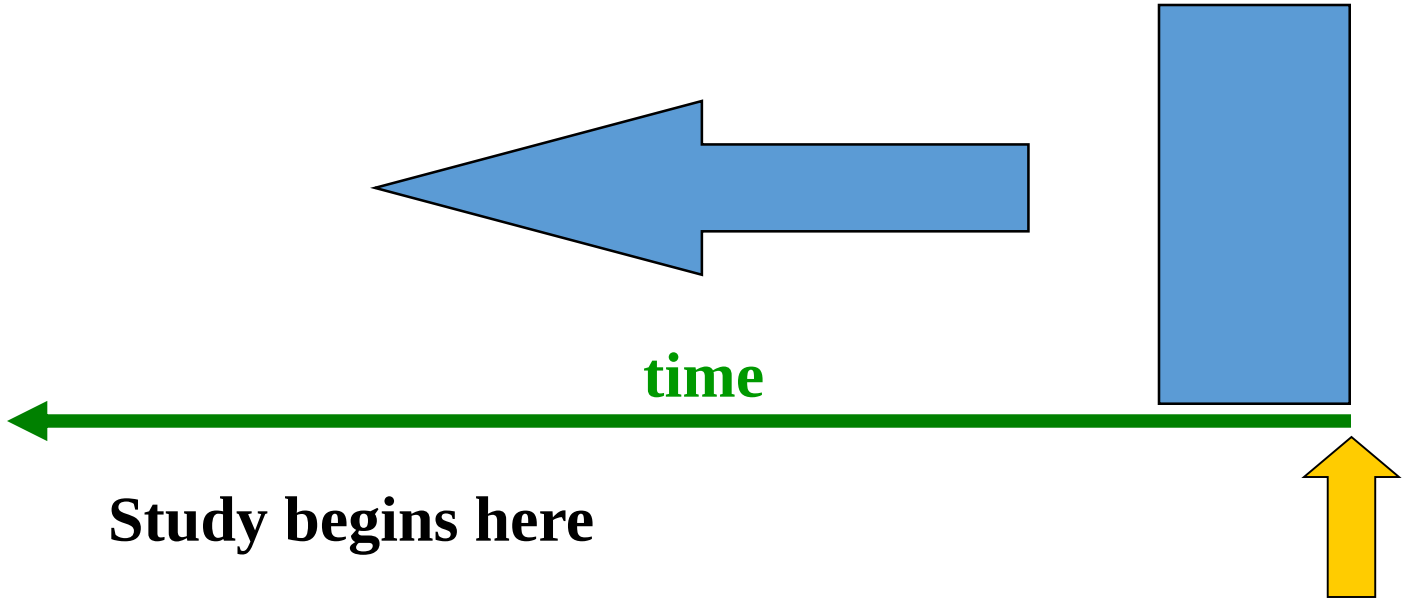
Timeframe of Studies

Prospective Study looks forward, looks to the future, examines future events, follows a condition, concern or disease into the future



Timeframe of Studies

Retrospective Study "to look back", looks back in time to study events that have already occurred



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:

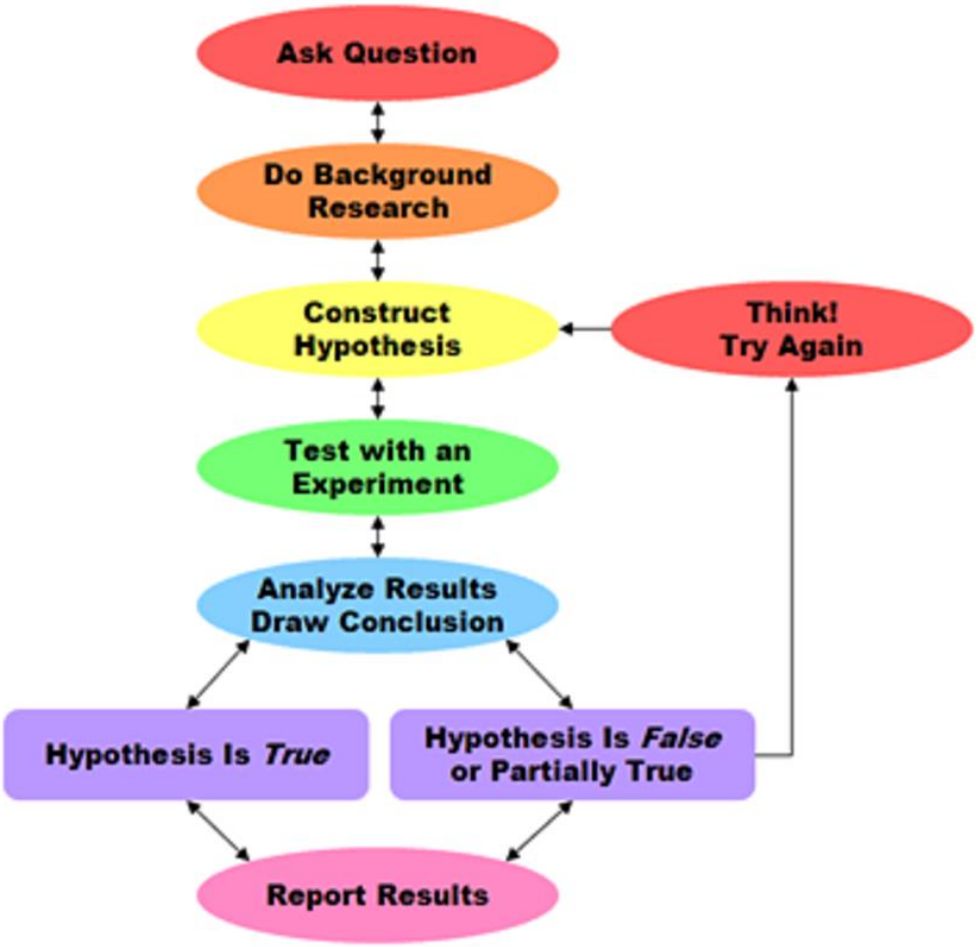
- Descriptive studies

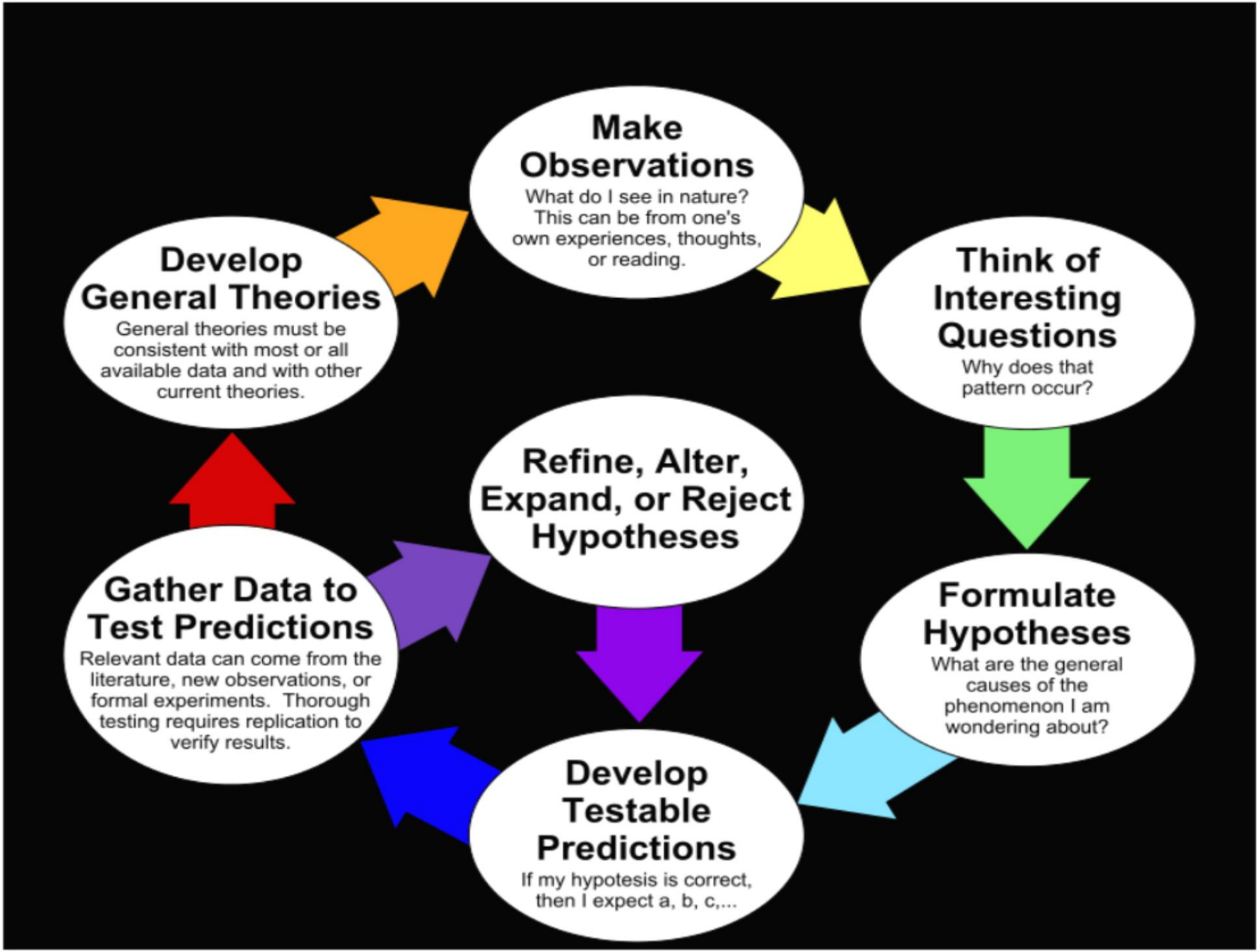
  describe occurrence of outcome

- Analytic studies

  describe **association** between exposure and outcome

Scientific Method

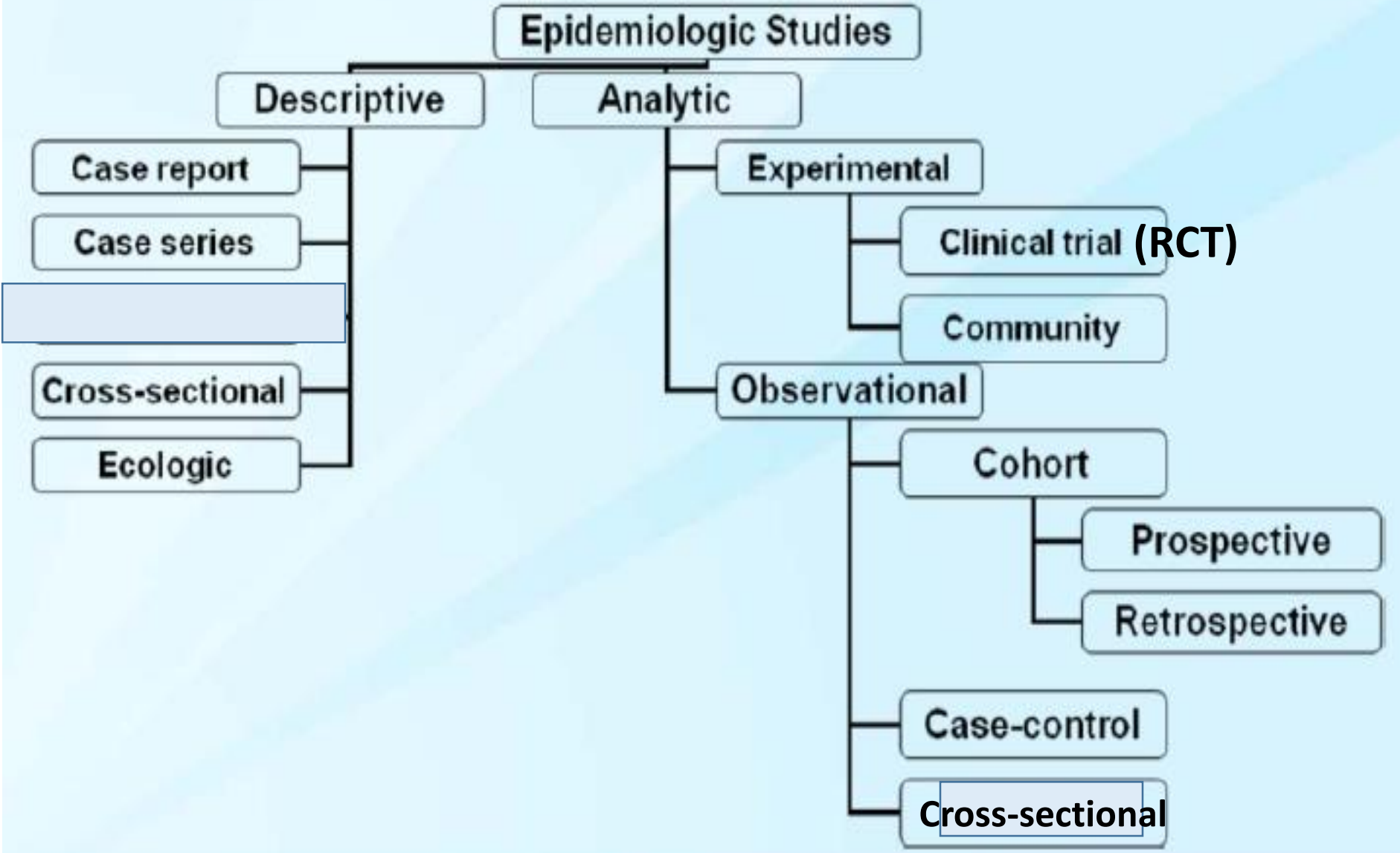


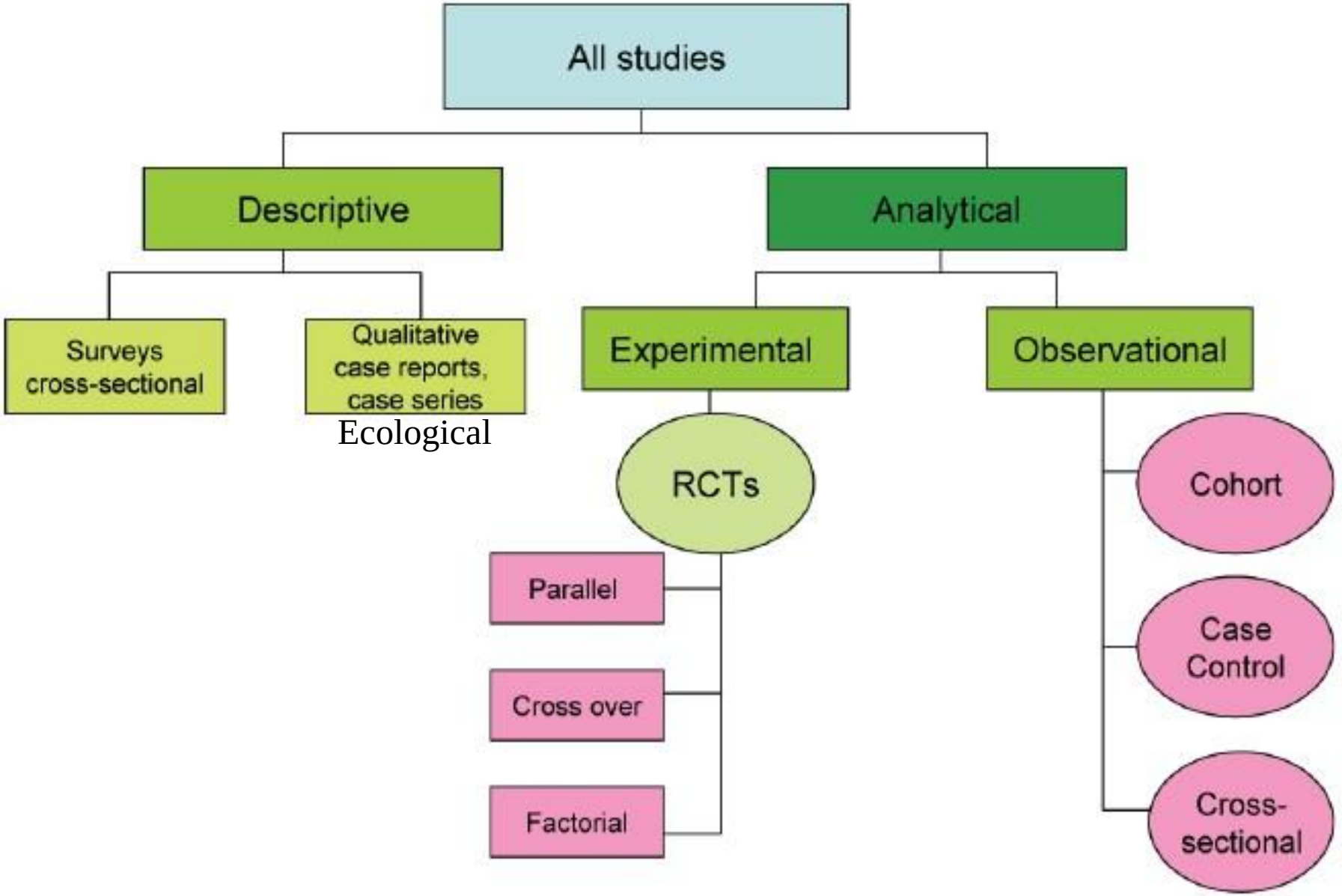

Make Observations
What do I see in nature? This can be from one's own experiences, thoughts, or reading.
Think of Interesting Questions
Why does that pattern occur?
Formulate Hypotheses
What are the general causes of the phenomenon I am wondering about?
Develop Testable Predictions
If my hypotesis is correct, then I expect a, b, c,...
Gather Data to Test Predictions
Relevant data can come from the literature, new observations, or formal experiments. Thorough testing requires replication to verify results.
Refine, Alter, Expand, or Reject Hypotheses
Develop General Theories
General theories must be consistent with most or all available data and with other current theories.

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




All studies
Descriptive
Analytical
Surveys cross-sectional
Qualitative case reports, case series
Ecological
Experimental
Observational
RCTs
Parallel
Cross over
Factorial
Cohort
Case Control
Cross-sectional

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 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

3. Ecological Studies (correlation study)

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

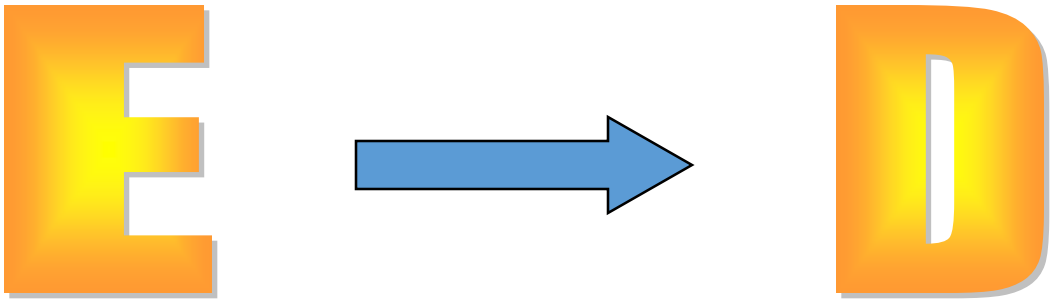
- It involves the collection of events over a defined population base and by the use of denominator data to determine rates.

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**

Basic Questions in Analytic Epidemiology

To prevent and control diseases ……
What is the exposure?
Who are the exposed?
What are the potential health effects?

- ❑ Generate a hypothesis about the relationship between exposure and effect, and then test this hypothesis.
- ❑ Study designs….. direct how this whole investigation is conducted.

Analytical Studies (testing hypothesis)

Observational Studies

- Cross-sectional
- Case-control
- Cohort

Experimental Studies

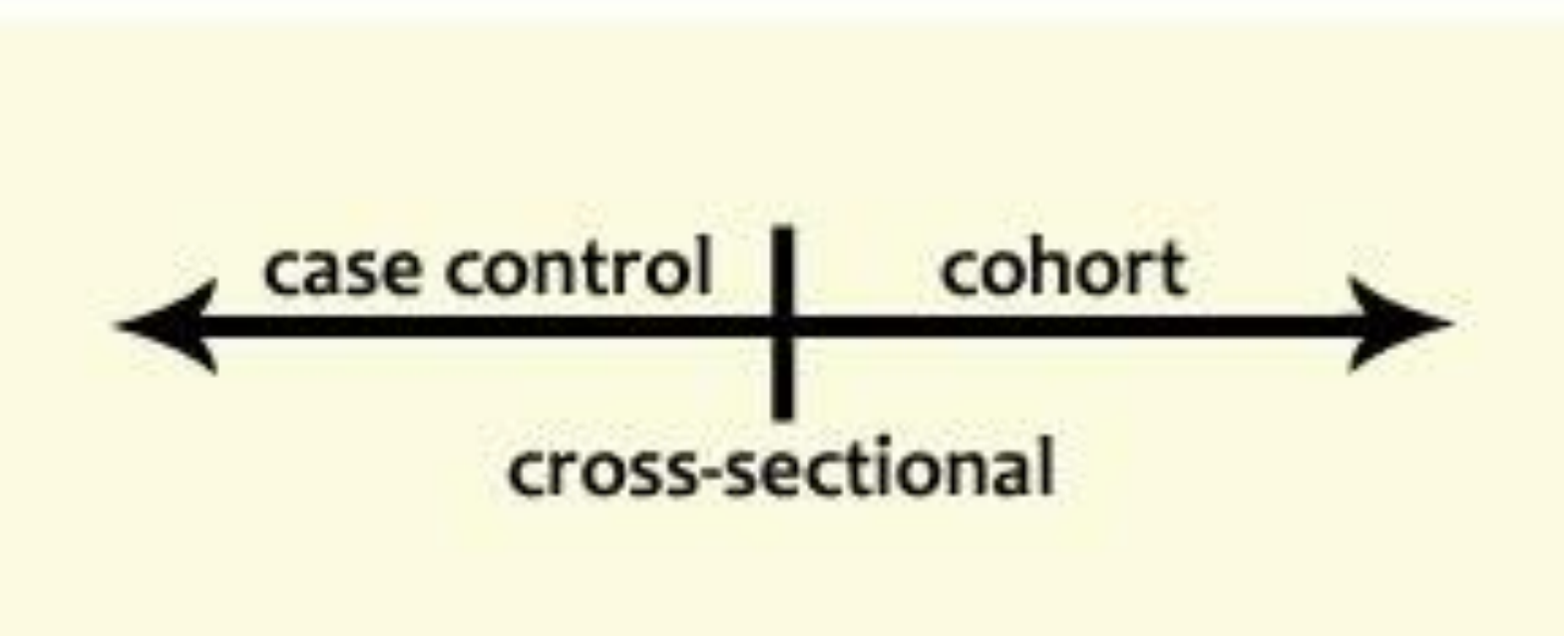
- Randomized controlled clinical trials
- Community trials

Observational Studies

non-experimental study designs

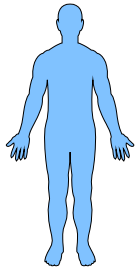
- Observational because there is no individual intervention
- Treatment and exposures occur in a “non-controlled” environment
- Individuals can be observed prospectively, retrospectively, or currently

Observational Studies

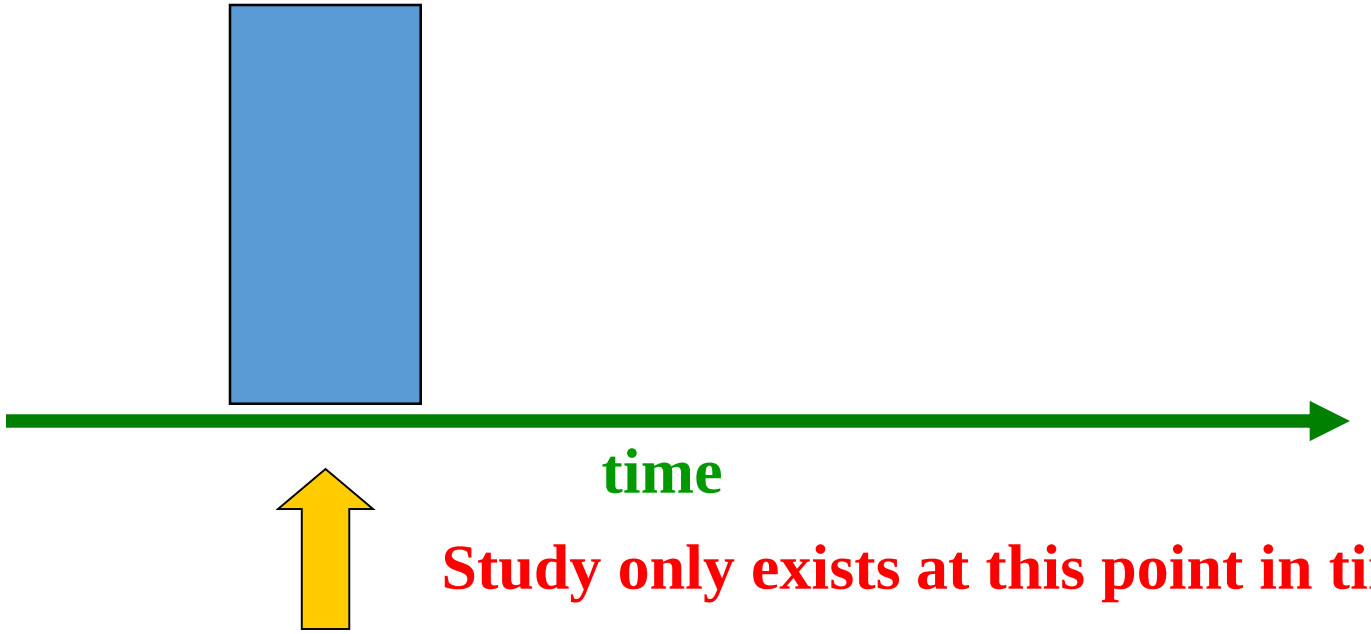


http://www.medbullets.com/step1-stats/1001/types-of-studies

Cross-sectional studies



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



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 which 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.

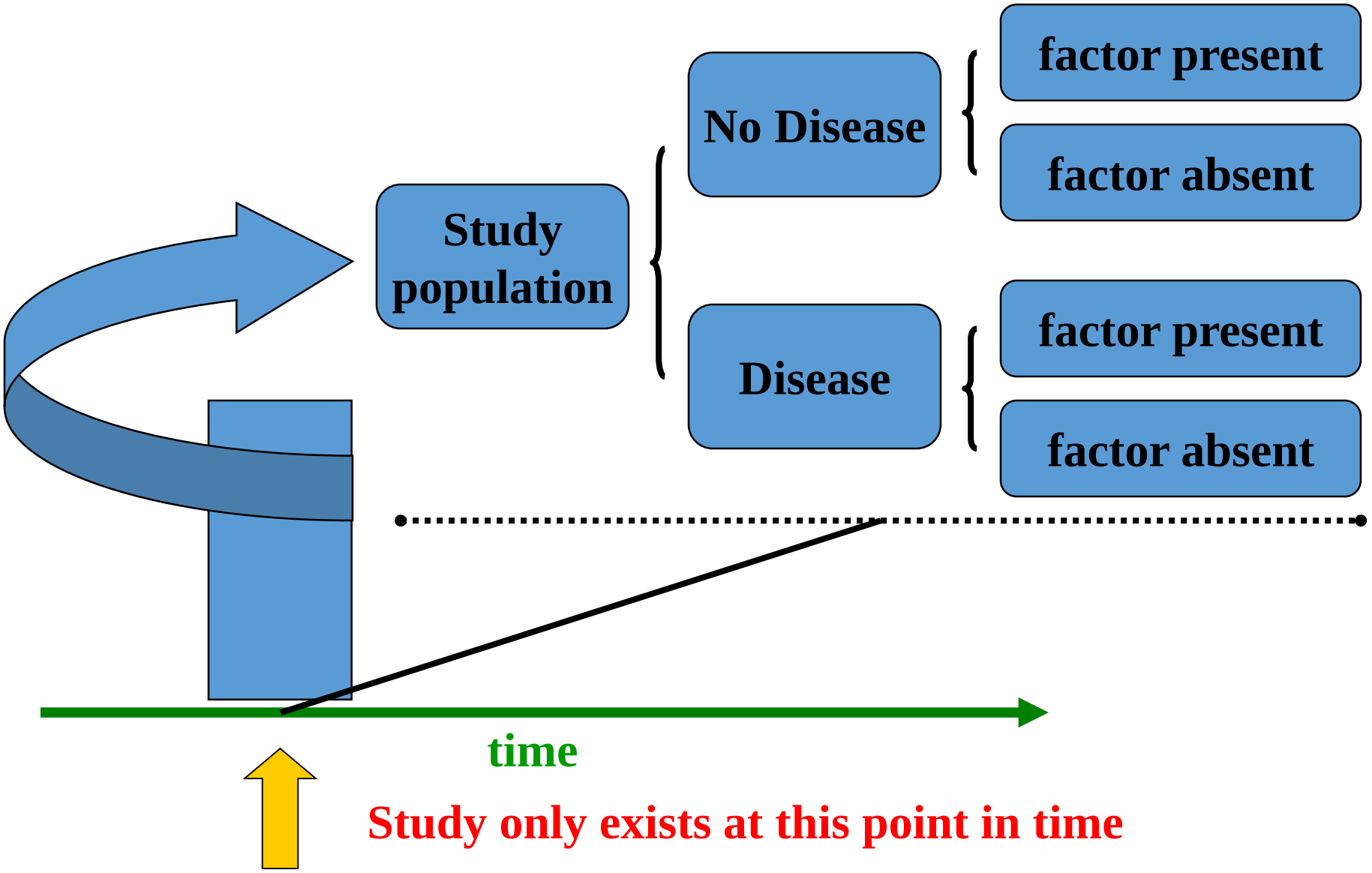
e.g. What is the prevalence of diabetes in Jordan?
A survey of asthma among animal handlers
A survey of dietary habits among university students.

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 of people like knowledge, 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
- 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 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 a problem.
- Least useful in establishing causation.

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 types of design.

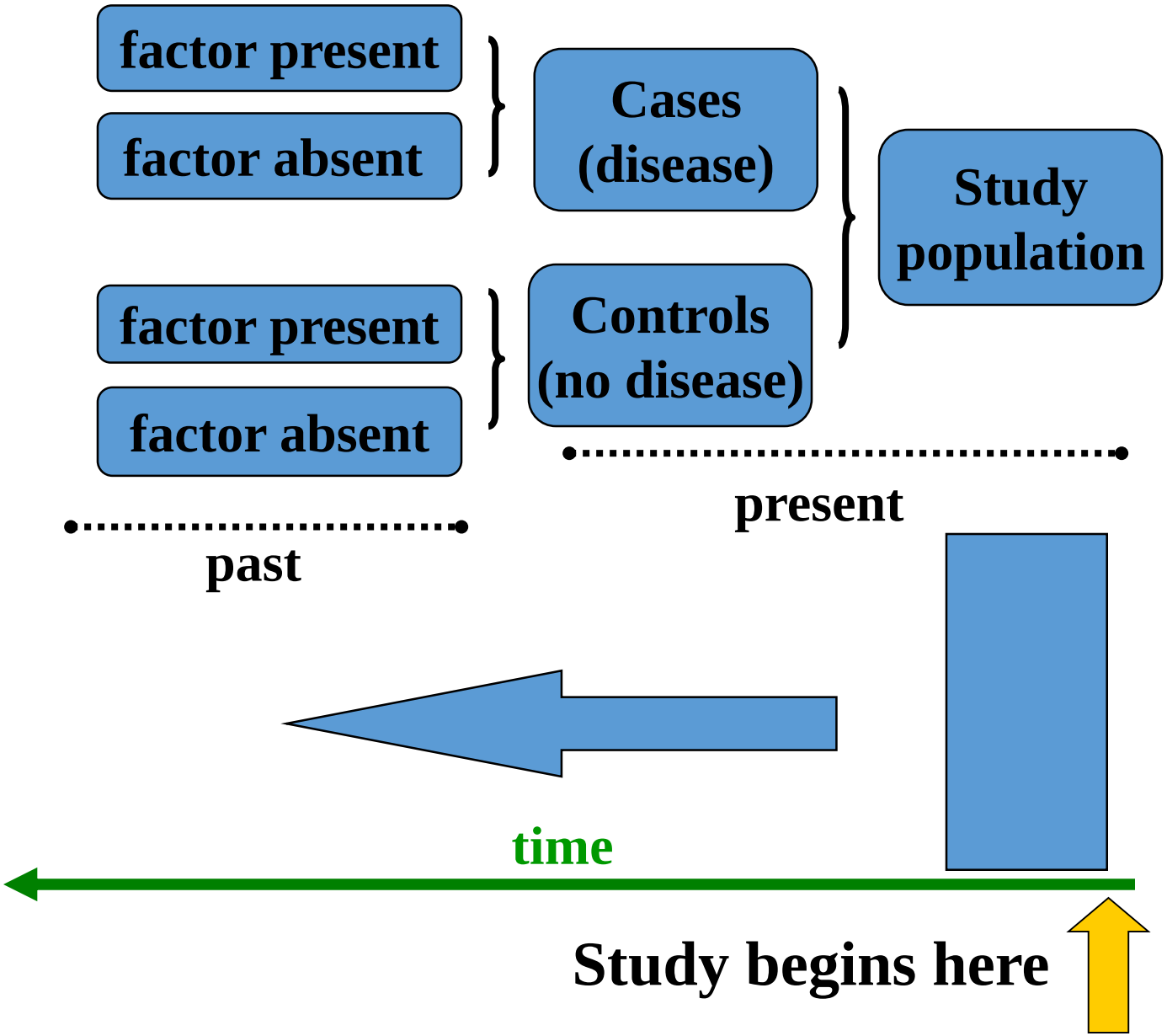
Case-Control Study Design

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 fo 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 outcomes are 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 aetiological 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. Inappropriate when disease outcome for a specific exposure is not known at start of study.
2. Selection of an appropriate control group may be difficult.
3. Inefficient for evaluation of rare exposure
4. Difficult to establish temporal sequence
5. Determining exposure will often rely on memory, leading to bias (recall bias).
6. 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).

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

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

Cohort Study

Subjects are selected by exposure and followed to see development of disease

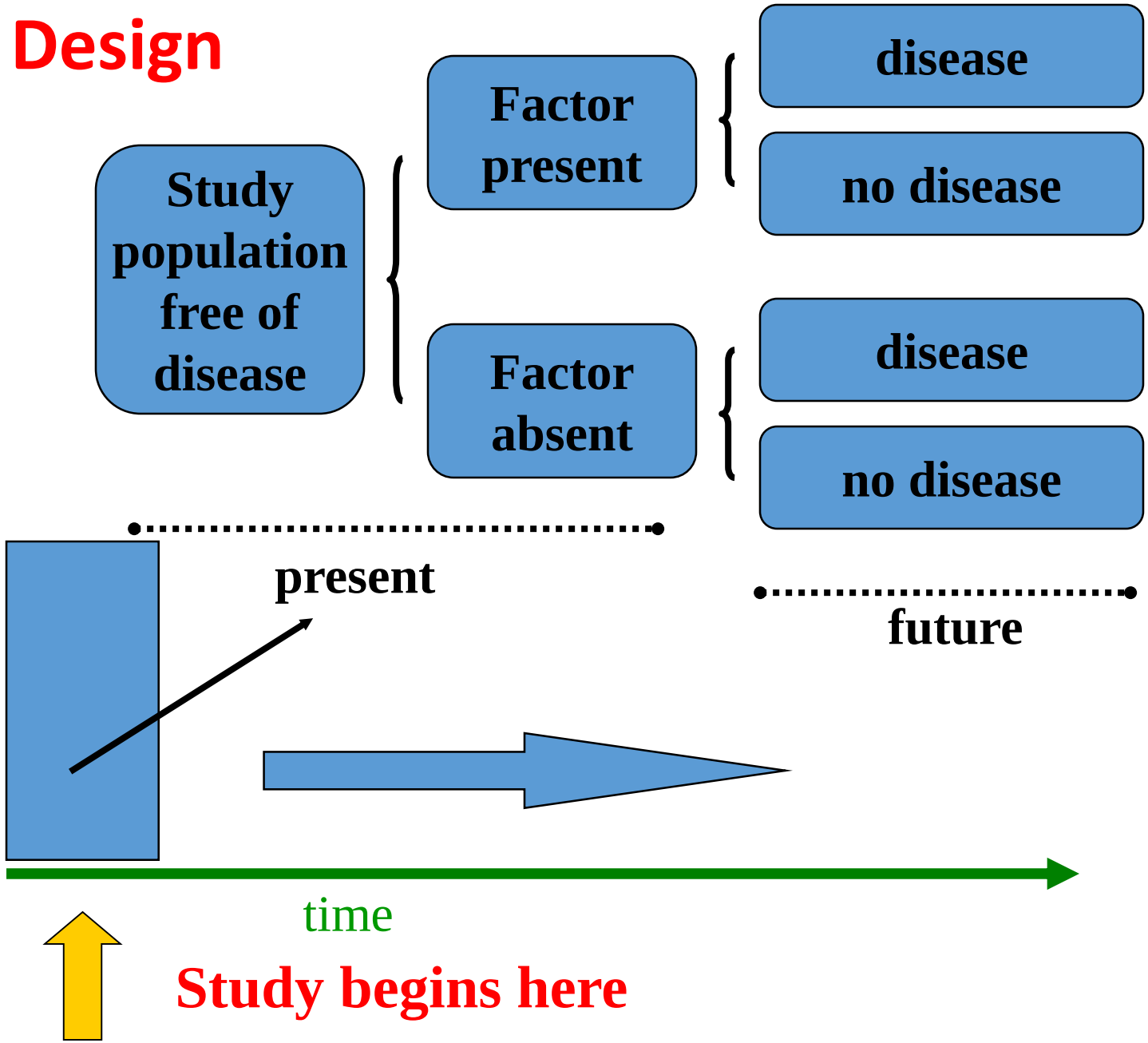
Cohort study is known by a variety of names: prospective study, longitudinal study, incidence study & forward looking study.

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 Design

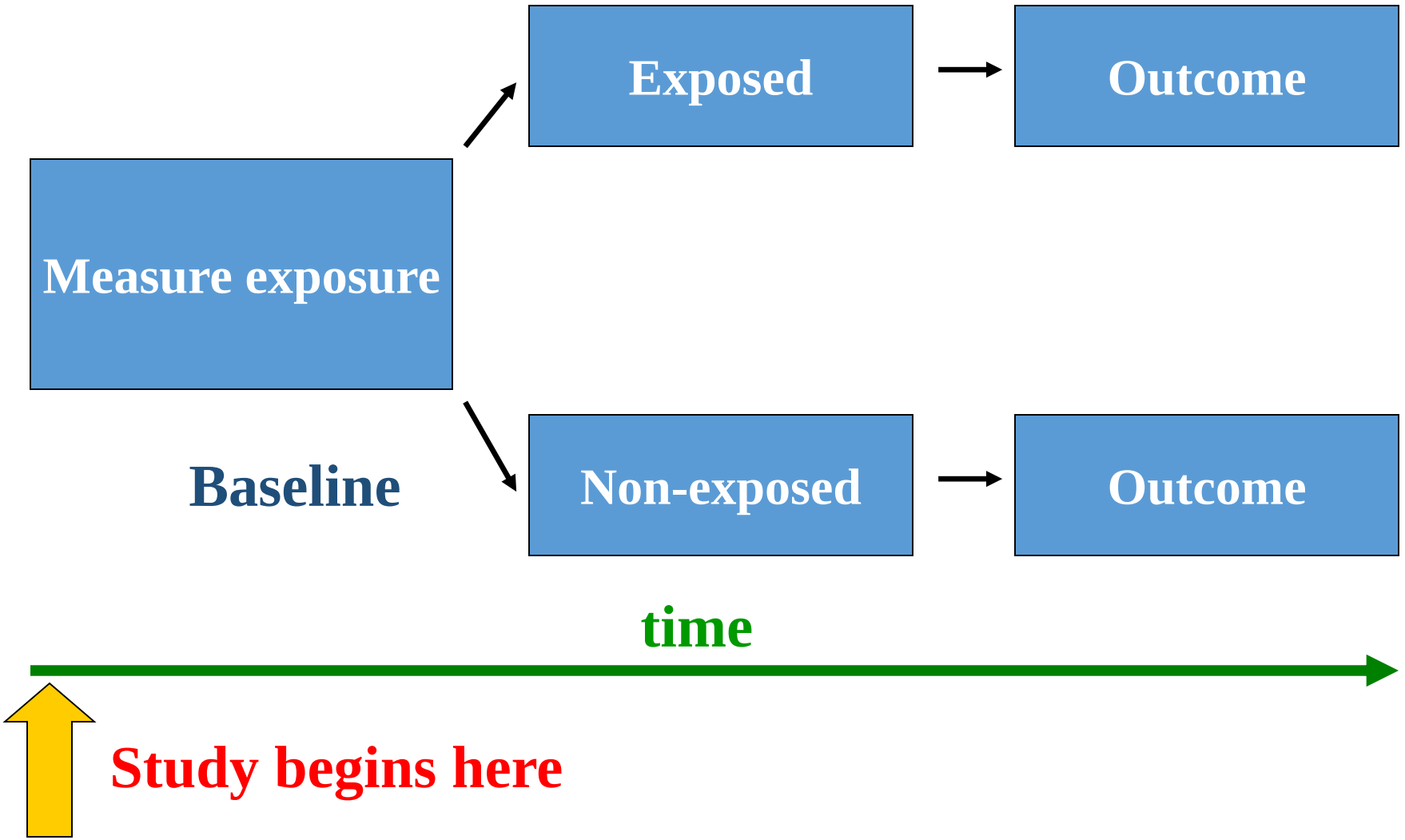


Cohort Study

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).

Prospective Cohort study



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

Experimental Studies (Intervention studies)

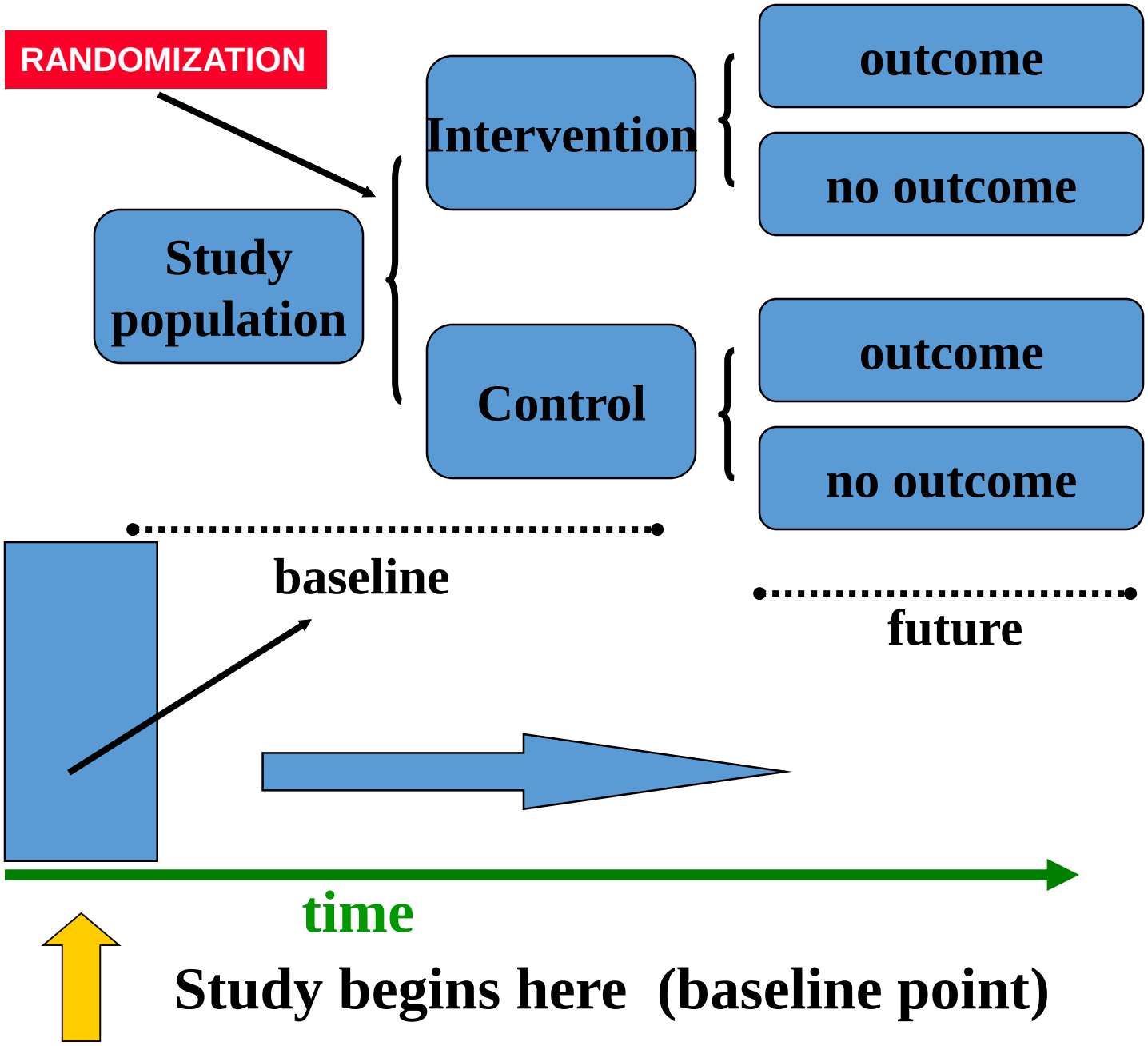
- In an experiment, we are interested in the effect or consequences of a new therapeutic treatment or procedure on an outcome.
- The subjects are allocated into a treatment group and a control group (old treatment or placebo).

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, cross-over studies.

Experimental Design



Experimental Design (Intervention studies)

If properly done, experimental studies can produce high quality data.

Thy are the **gold standard** study design (strongest, most robust).

The quality of "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 observation bias.

Placebo: an inert material indistinguishable from active treatment. Used to avoid Placebo effect: tendency to report favourable response regardless of physiological efficacy.

(Placebo is used as blinding procedure)

RCT (Randomized Controlled Trial)

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).

Randomized Controlled Trials (RCTs)

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

Randomized Controlled Trials

Disadvantages of RCTs:

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

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

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)

Decision Tree