

Fundamentals Biostatistics

Lecture 2: Study Design

Definition of study design

Definition: A study design is a specific plan or protocol for conducting the study, which allows the investigator to translate the conceptual hypothesis into an operational one.

Types of study designs:

- Qualitative
- Quantitative (Observational and Experimental)

Quantitative design

Quantitative design

- **Observational**: studies that do not involve any intervention or experiment.
- **Experimental**: studies that entail manipulation of the study factor (exposure) and randomization of subjects to treatment (exposure) groups

Observational design

A clinical case series is a descriptive medical study that analyzes a group of patients with similar characteristics or a particular disease or condition to identify common features and patterns. These studies are valuable for generating hypotheses, describing trends, and raising awareness of new or rare diseases or treatments, though they lack a control group and cannot establish causality or generalizable conclusions.

1. Clinical case-series

Clinical case-series is usually a coherent and consecutive set of cases of a disease (or similar problem) which derive from either the practice of one or more health care professionals or a defined health care setting, e.g. a hospital or family practice.

A case-series is, effectively, a register of cases.

Analyse cases together to learn about the disease.

Clinical case-series are of value in:

- Studying symptoms and signs
- Creating case definitions
- Clinical audit and research

A cross-sectional study is an observational research method that collects data from a population at a single point in time to assess the prevalence of conditions or traits, identify health determinants, and establish associations between variables

2. Cross-sectional Studies(Community health ,surveys)

Characteristics: detects point prevalence; relative conditions; allows for stratification

Merits: feasible; quick; economic; allows study of several diseases / exposures; health planning and priority setting of health problems

Limitations: temporal ambiguity (cannot determine whether the exposure preceded outcome); possible measurement error; not suitable for rare conditions; liable to survivor bias.

3. Case-Control Studies

Characteristics: two source populations; assumption that non-cases are representative of the source population of cases.

Merits: least expensive; least time-consuming; suitable for study of rare diseases (especially NCDs)

Limitations: not suitable for rare exposures; liable to selection bias and recall bias; not suitable for calculation of frequency measures.

A case-control study is an observational study that compares two groups: individuals with a specific disease or condition (cases) and similar individuals without the condition (controls). Researchers look back in time (retrospective) to compare past exposures and lifestyle histories between the two groups, identifying factors that may be associated with the health outcome. These studies are useful for rare diseases but are prone to biases like recall bias.

A cohort study is an observational research design where a group of individuals (a cohort) who share a common characteristic is followed over time to determine how a specific exposure is associated with a health outcome.

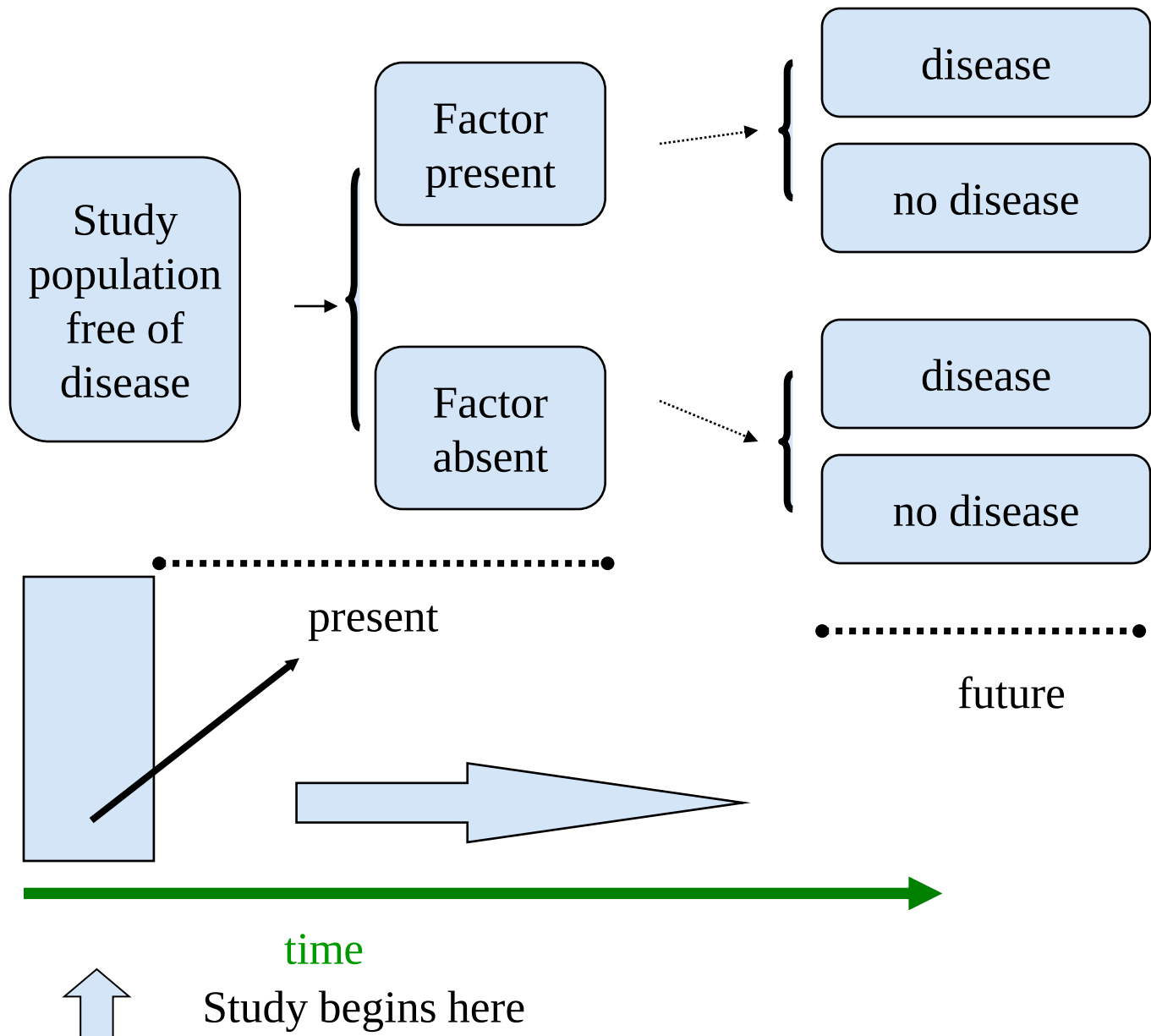
4. Cohort Studies

Characteristics: follow-up period (prospective; retrospective)

Merits: no temporal ambiguity; several outcomes could be studied at the same time; suitable for incidence estimation

Limitations (of prospective type): expensive; time-consuming; inefficient for rare diseases; may not be feasible

Cohort Design



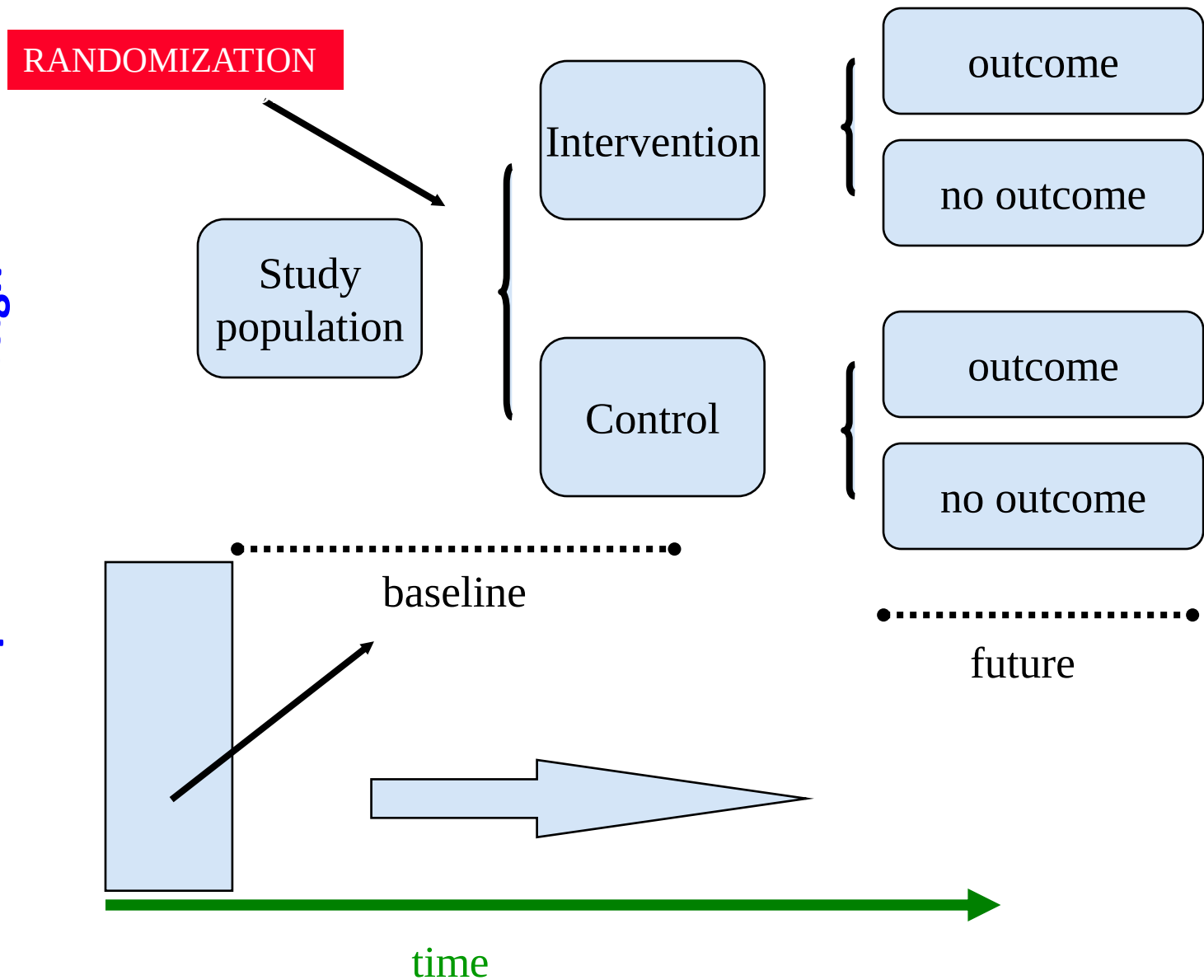
Experimental Designs

Experimental Study Design

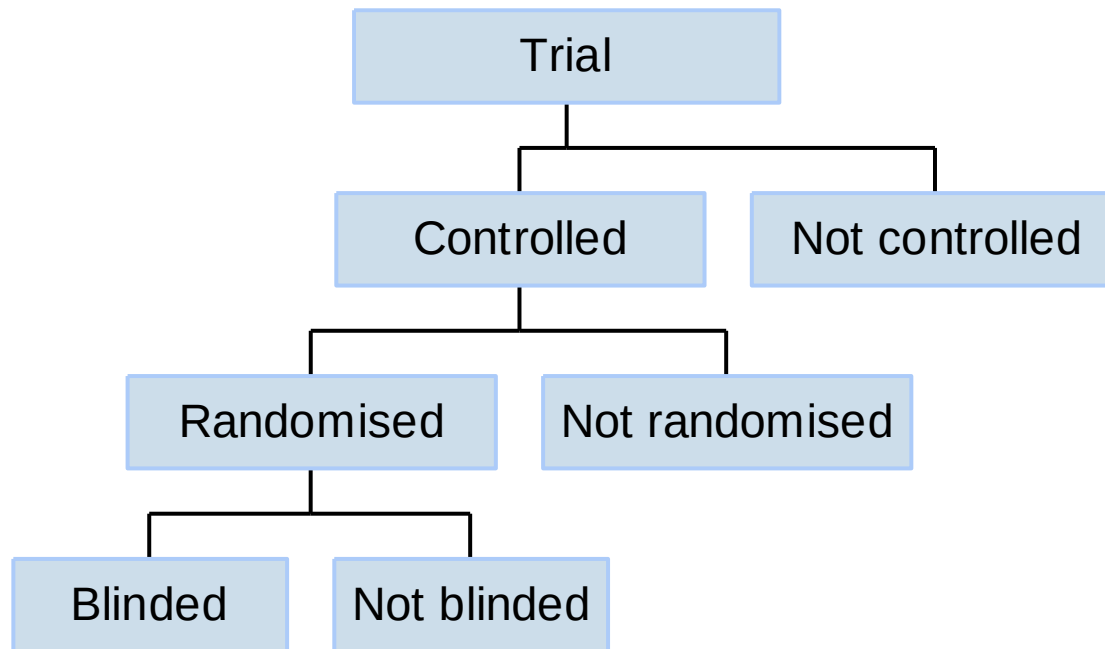
A study in which a population is selected for a planned trial of a regimen, whose effects are measured by comparing the outcome of the regimen in the experimental group versus the outcome of another regimen in the control group.

Such designs are differentiated from observational designs by the fact that there is manipulation of the study factor (exposure), and randomization (random allocation) of subjects to treatment (exposure) groups.

Experimental Design



Types of trials



- Blinded Randomized Controlled Trial is the gold standard design
- It Provides the strongest evidence

Choice of Study Design

Depends on:

- Research Questions and goals.
- Time and Funds
- Status of existent knowledge
- Occurrence of disease
- Nature and availability of information
- Available resources