Study Designs in Epidemiology

Case-control, Cohort and Experimental Designs

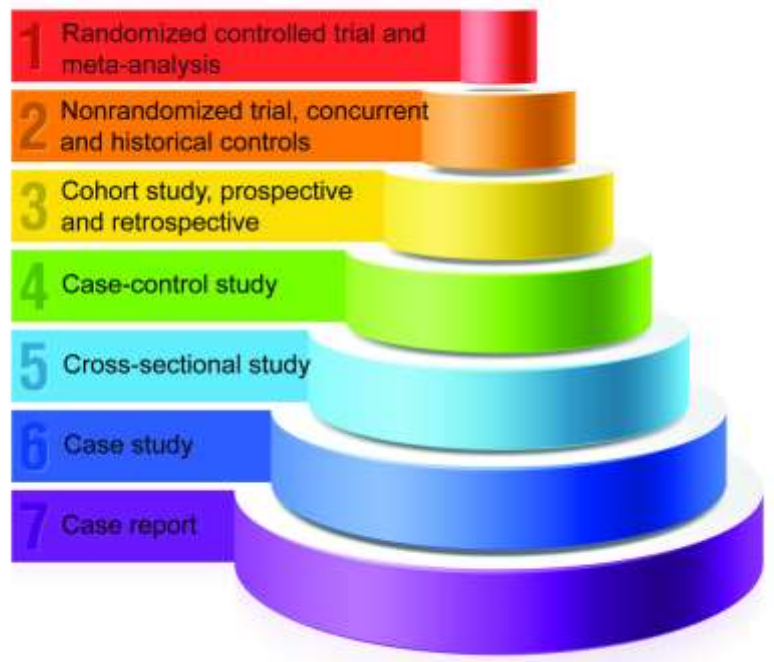

Figure. Hierarchy of Research Design

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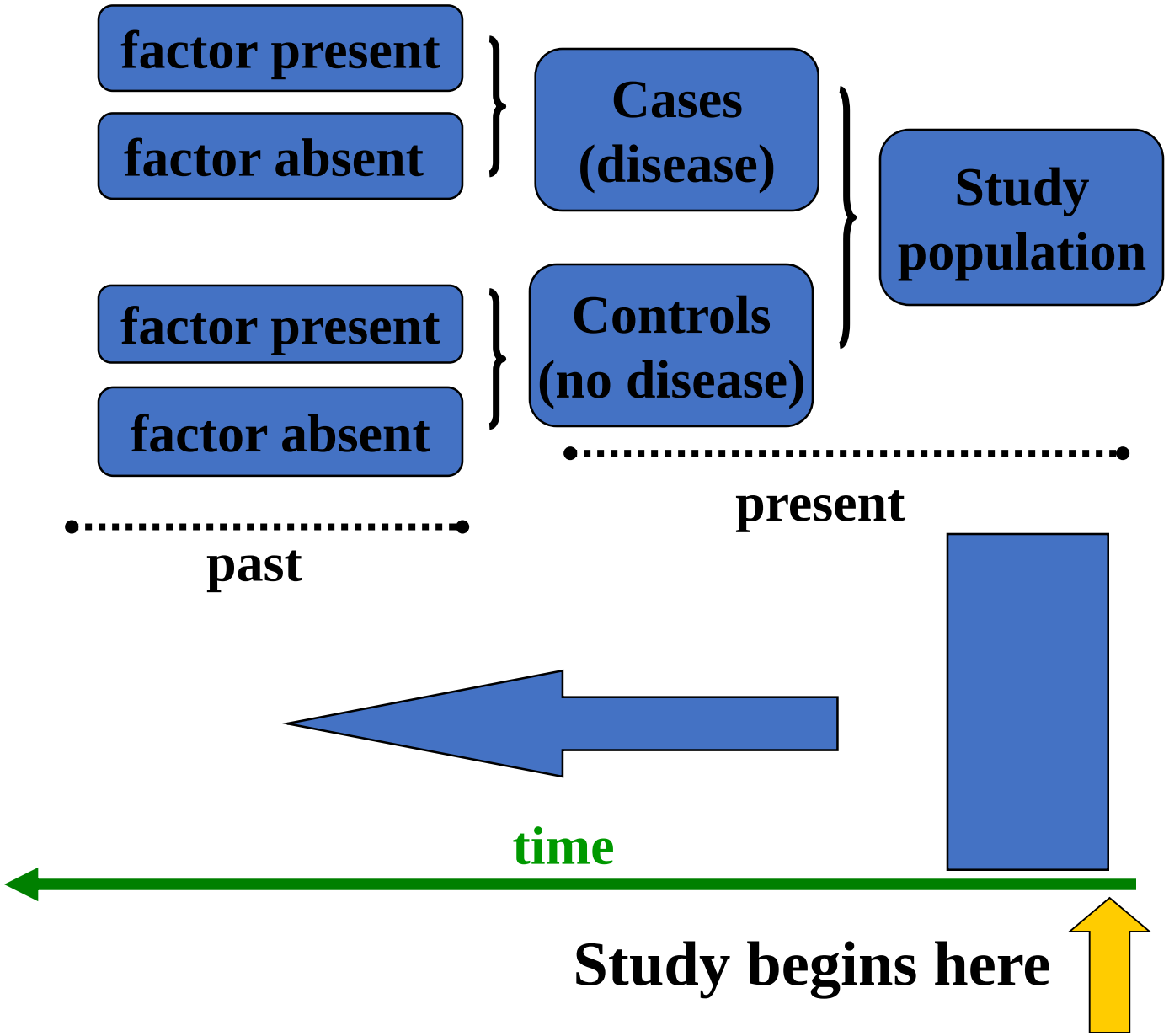
Case-Control Study Design

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

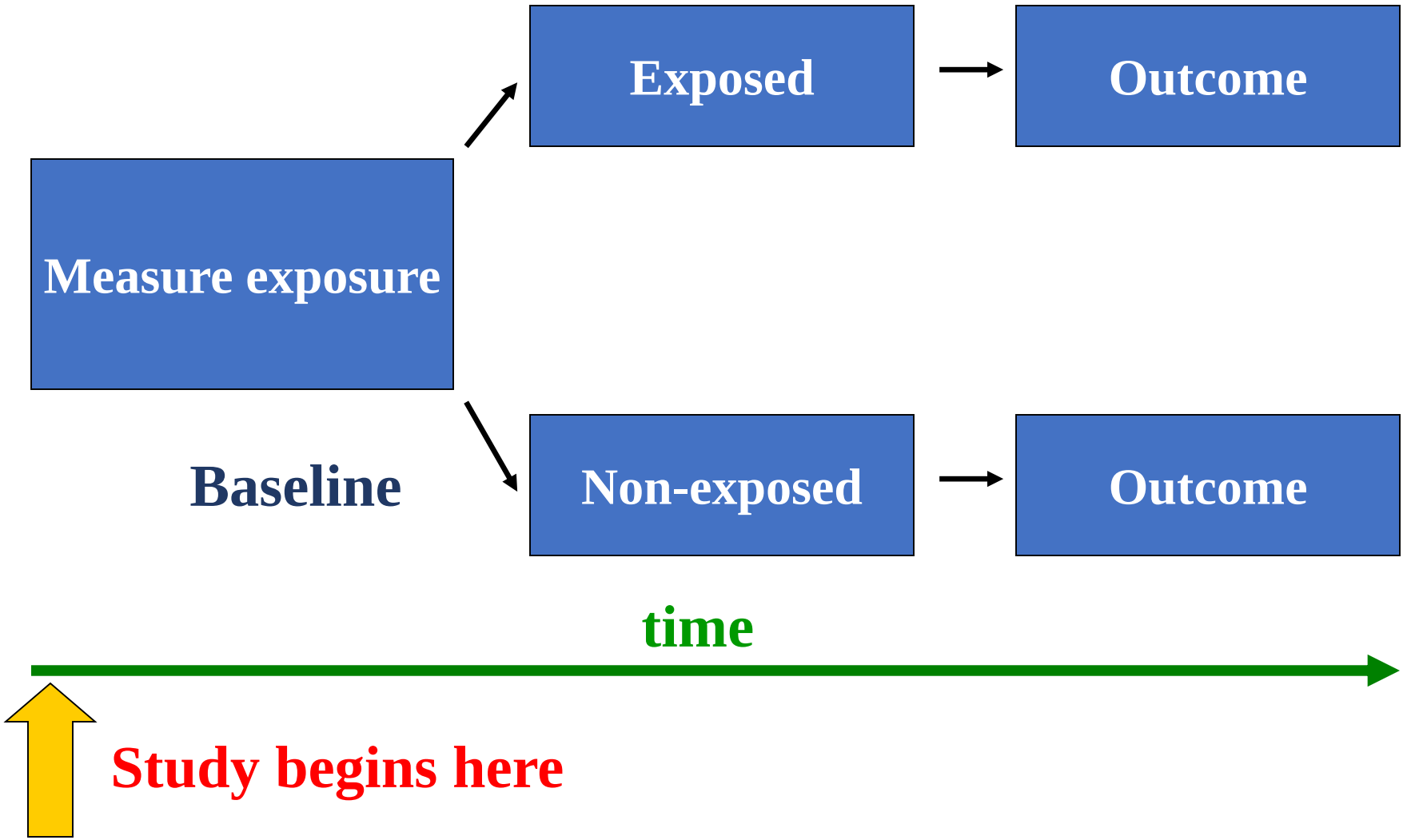
In a COHORT STUDY, a group of individuals that is exposed to a risk factor (study group) is compared with a group of individuals not exposed to the risk factor (control group)….and all followed up to monitor occurrence of disease.

- 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?

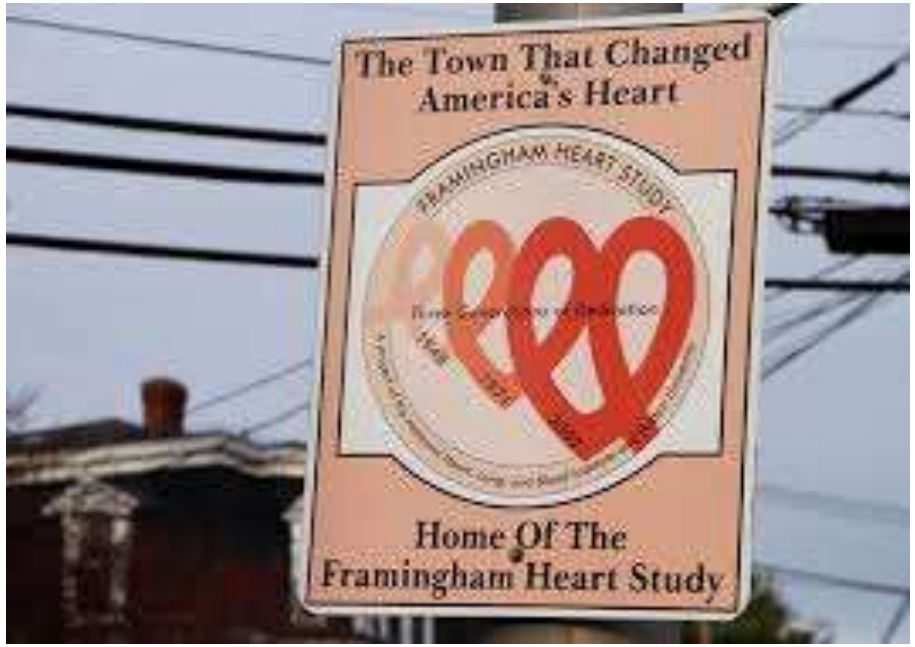
When did it start? Where?

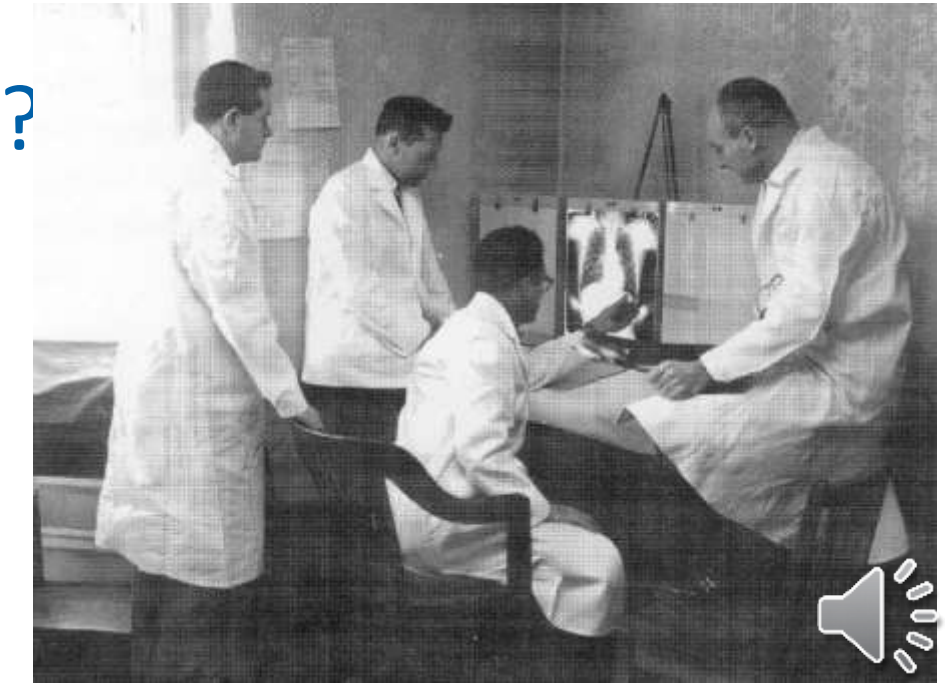
What was the disease studied?

What are the most important findings?

How many people participated?

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.

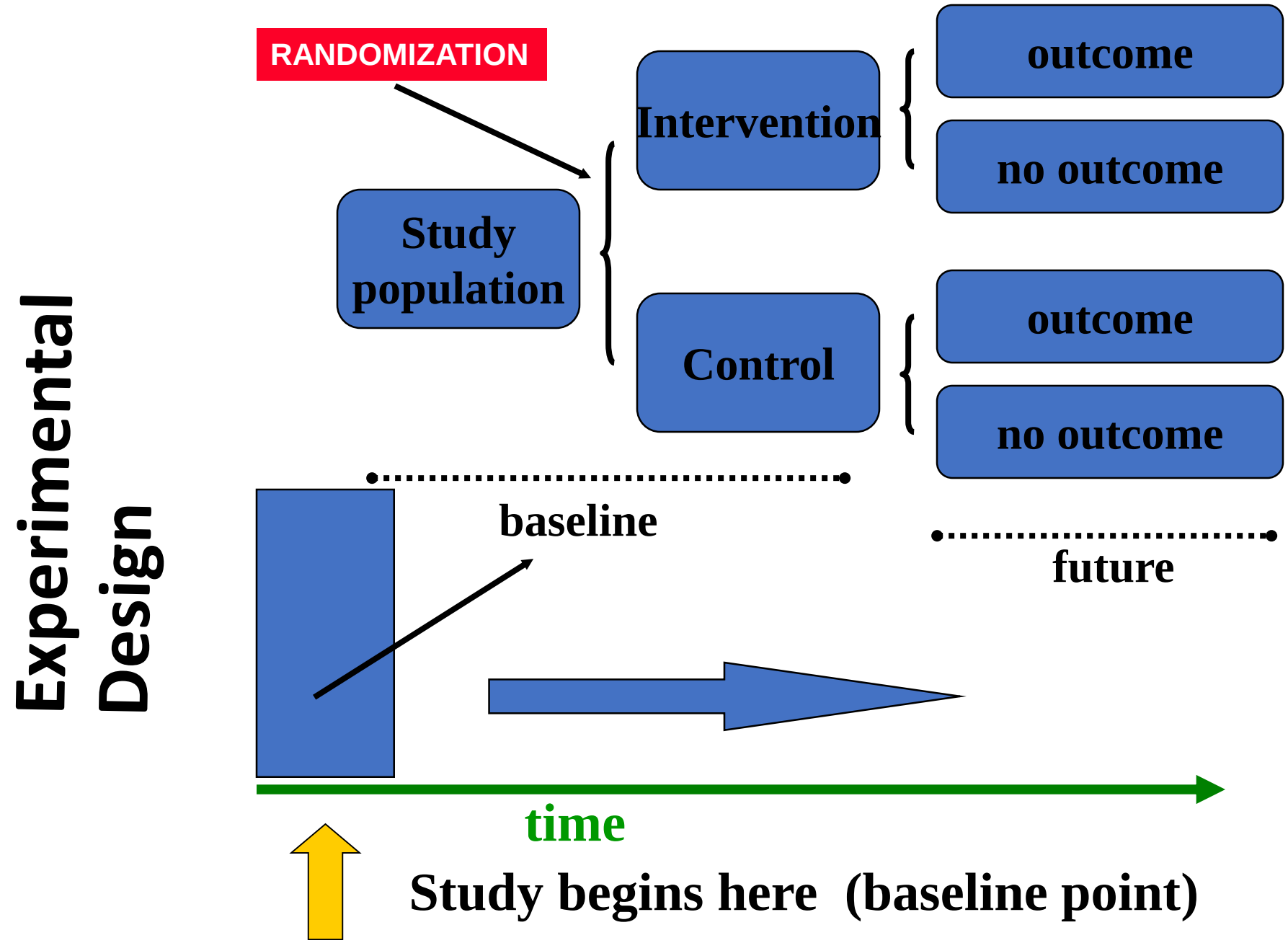
RCT is a clinical trial that is well-designed (controlled and randomized).

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 occurred naturally : e.g. Increase in mental disorders following an earthquake.

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