Community Medicine

Summary

Slide # 12

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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.

• Society will be healthier

• Society can save money on health care budgets

• It will improve life expectancy

• It will improve the economy

Types of Epidemiologic Study Designs

I. Based on objective/focus/research question:

1. Descriptive studies

Describe: who, when, where & how many

Describe occurrence of outcome

2. Analytic studies

Analyze: How and why

Describe association between exposure and outcome

- Descriptive studies examine the frequency to which diseases occur. Analytic studies evaluate the relationship of disease to different exposures

II. Based on the role of the investigator

1. Observational studies

- The investigator observes nature
- No intervention

2. Intervention/Experimental studies

- Investigator intervenes
- He has a control over the situation

III. Based on timing:

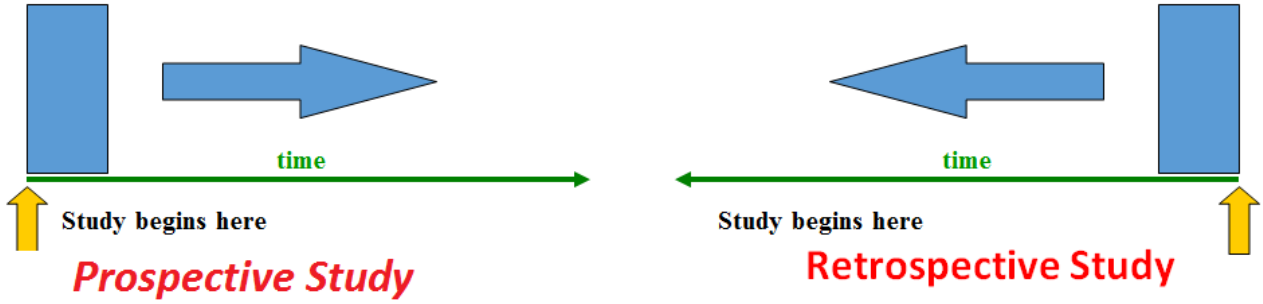
1. One-time (one-spot) studies

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

2. Longitudinal (Follow-up) studies

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

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



1. Prospective

Conducted forward in time, looks forward, looks to the future, examines future events, and follows a condition, concern or disease into the future. With a prospective study one starts with cohorts of well individuals, and we wait until events occurs.

2. Retrospective

Conducted backward in time "to look back", looks back in time to study events that have already occurred. Looking backward is often difficult because of recall bias, however, the case control studies are very inexpensive in comparison with prospective studies.

V. Based on type of data they generate

1. Qualitative studies

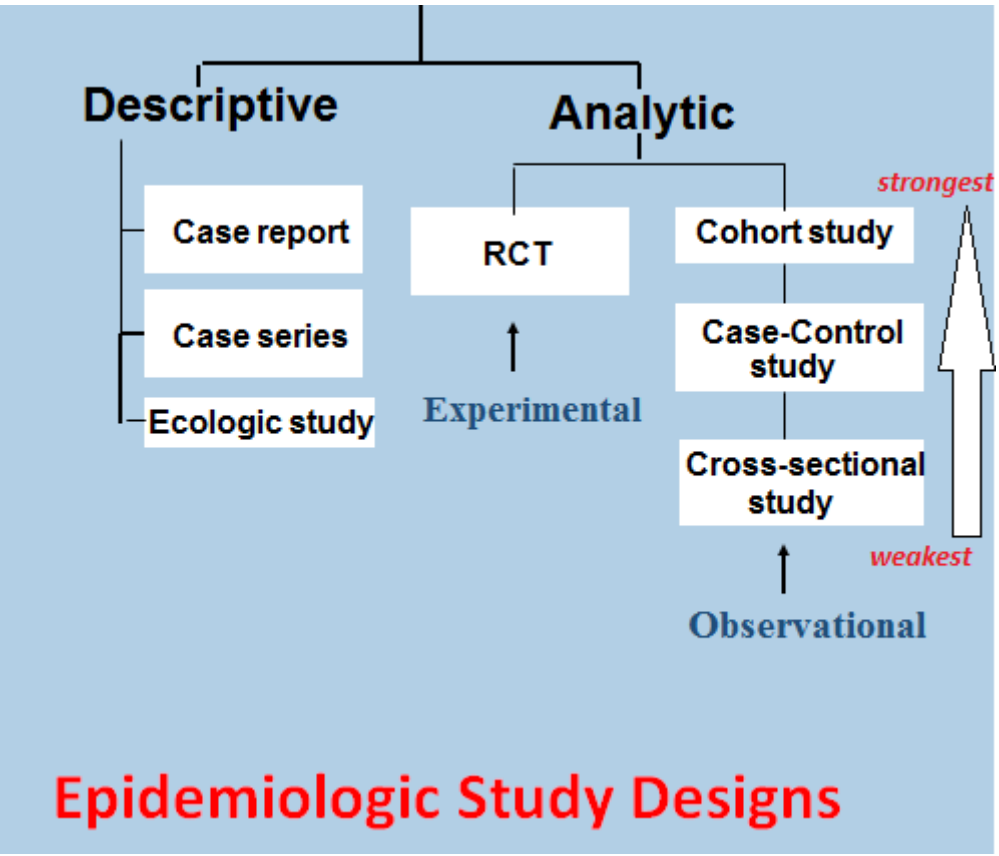
- Generate contextual data
- Also called exploratory studies

2. Quantitative studies

- Generate numerical data
- Also called explanatory studies

VII. Standard classification

1. Cross-sectional studies
2. Case-control studies
3. Cohort studies
4. Experimental studies



FORMULATING 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.

Now, study designs types will be discussed.

Descriptive Studies (generating hypothesis)

- Descriptive studies are usually the first phase of an epidemiological investigation. These describe the frequency or characteristics of events.
- These studies are concerned with observing the distribution of disease or health – related characteristics in human populations.

TIME "When"	**PLACE** "Where"	**PERSON** "Who"
Year, Season	*Climatic zones*	*Age, Birth order*
Month, Week	*Country, region*	*Sex, Family size*
Day, Hour of onset	*Urban/ rural / Local community*	*Marital State, Height, Weight*
Duration	*Towns, Cities, Institutions*	*Occupation, Social status, Education, Blood pressure, Blood cholesterol, Personal habits*

- Such studies basically ask the questions of what, who, where, and when.
- We will discuss 3 descriptive study designs:

1- **Case Reports**: Detailed presentation of a single case or handful of cases. Generally reports a new or unique finding e.g. previous undescribed disease; unexpected link between diseases; unexpected new therapeutic effect; adverse events. Case reports are in many ways “sentinel events” which can lead to testable hypotheses

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 also provide suggestive evidence many times leading to more extensive testing.

   - Advantages

Useful for hypothesis generation

Informative for very rare diseases with few established risk factors

- Disadvantages

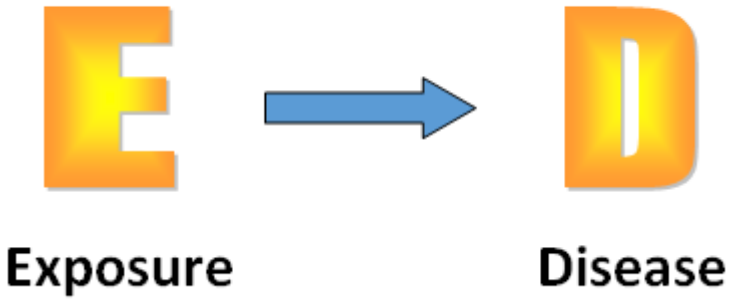
Cannot study cause and effect relationships

Cannot assess disease frequency

3- **Ecologic study**: it 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 (A fallacy is the use of invalid or otherwise faulty reasoning, or "wrong moves" in the construction of an argument).

Analytical studies (Are exposure and disease linked?)

These are Basic designs in epidemiology examine if exposures are correlated with 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.

In order to examine the link of exposure to disease, there needs to be standardized evaluation of exposure, as well as disease.

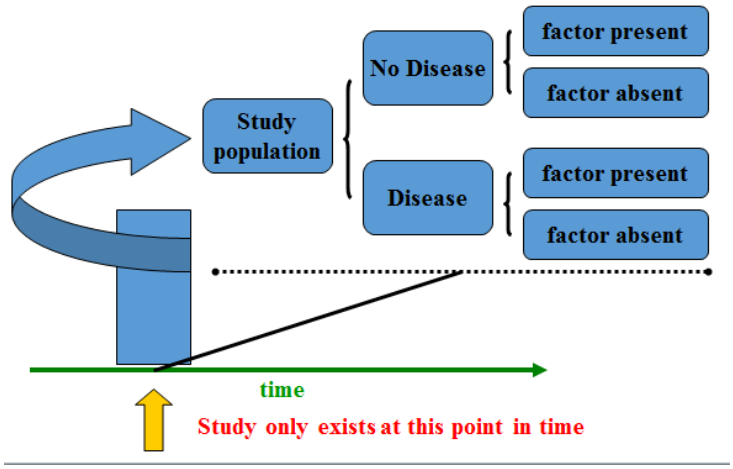
Analytical studies are divided into observational and experimental:

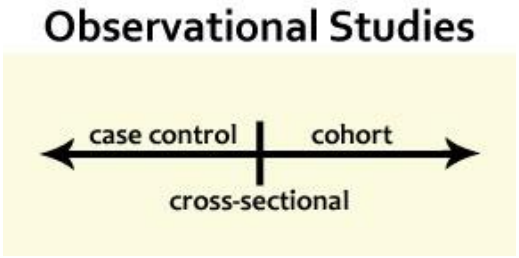
Observational Studies	Experimental Studies
• Cross-sectional • Case-control • Cohort	• Randomized controlled clinical trials • Community trials

Observational studies:

Non-experimental study designs. They are called observational because there is no individual intervention; Treatment and exposures occur in a "non-controlled" environment. Individuals can be observed prospectively, retrospectively, or currently.

1- **Cross-sectional studies**





This study is an "observational" design that surveys exposures and disease status at a single point in time (a cross-section of the population; it is like a caption). Cross sectional studies are some of the first studies completed because of ease and low cost.

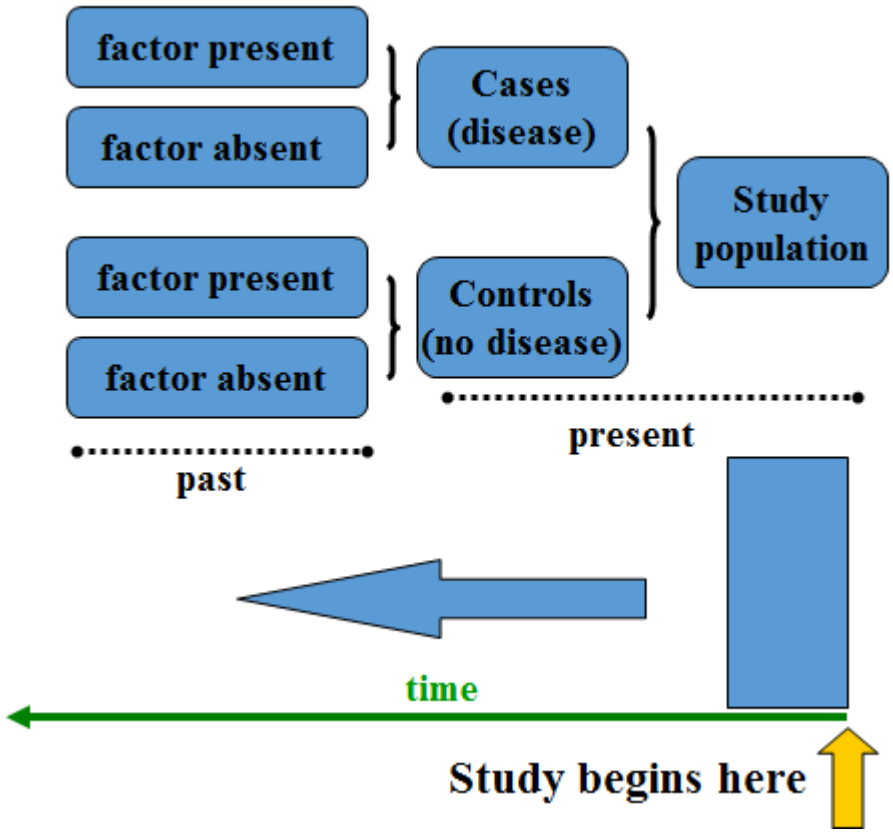
Cross sectional studies are the simplest form of observational studies. It is often used to study conditions that are relatively frequent with long duration of expression (nonfatal, chronic conditions). It measures prevalence, not incidence of disease. Example: community surveys. Note that this design is not suitable for studying rare or highly fatal diseases or a disease with short duration of expression; because cross-sectional studies involve point prevalence, not incidence, and for very infrequent diseases they are of limited utility. Remember that prevalence does not include incidence.

<u>Advantages of cross-sectional studies</u>

- 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.



<u>Disadvantages</u>

- 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 "because of this, this design is sometimes referred to as a descriptive study."

2- **Case-Control Studies**

It is an "observational" design comparing exposures in disease cases vs. healthy controls from the same population. Exposure data are collected retrospectively. This is the 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. Case-control studies in epidemiology are the most used type of study design.

<u>SELECTION OF CONTROLS</u>

- 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.

  Selection of an appropriate control group is an important pre requisite, because we will be making *comparison* with controls. Remember that comparisons are crucial in epidemiological studies.

  Strengths:

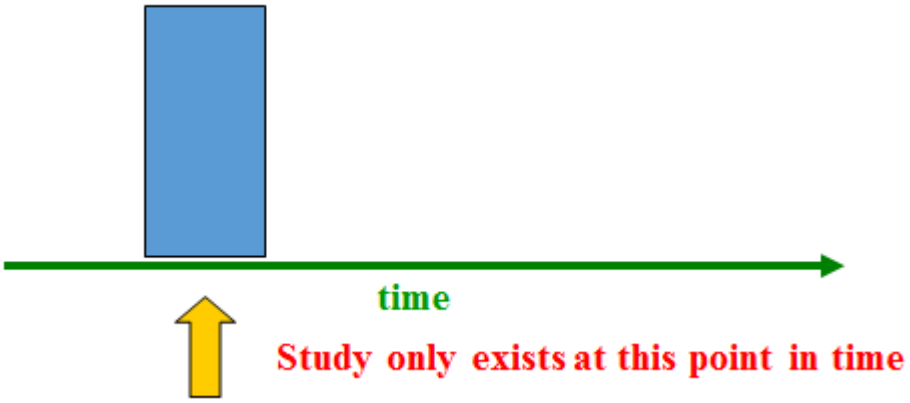
  - Less expensive and less time consuming
  - Efficient for studying rare *diseases*
  - Allows the study of several different etiological factors for one disease.
  - No attrition problems "**attrition** is ratios regarding the loss of participants during an experiment"
  - Ethical problems minimal (no risk to participants)

  Case control studies provide low cost answers to health questions.

  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* "but it is good for rare *diseases*"
  4. Difficult to establish temporal sequence
  5. Determining exposure will often rely on memory, leading to bias.
  6. We cannot measure incidence, we can only estimate the relative risk.

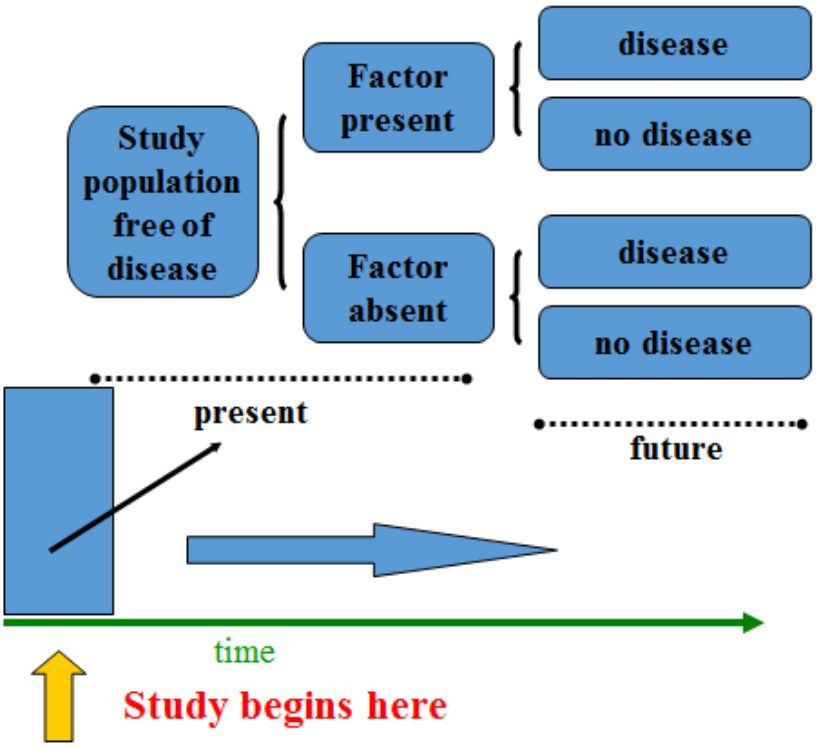
3- **Cohort Study**



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. A cohort studies follows a cohort of individuals who do not have disease, and then identified over time those individuals who have an outcome.

Case-control studies are perhaps the most frequent form of analytic study design. These designs are very good for events that are rare in occurrence.

However, there are some situations where cohort study designs would be appropriate in the field.



The study begins by assessing baseline levels of the exposure and other variables. Study subjects are then followed on a regular basis to identify the outcome. The frequency of outcomes is tested between persons who had exposure to the possible risk factor at baseline and persons with no exposure.

It is an "observational" design comparing individuals with a known risk factor or exposure with others without the risk factor or exposure.

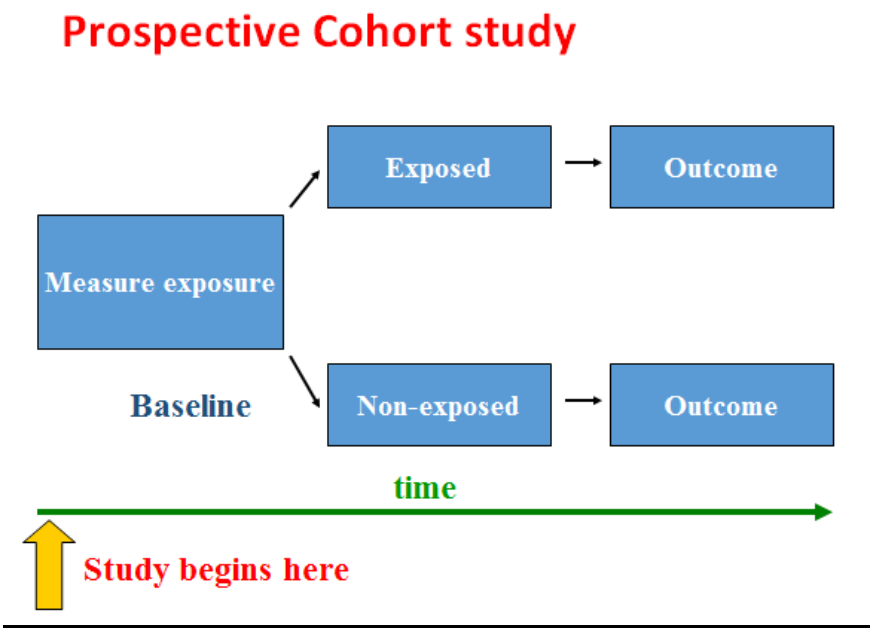
- Looking for a difference in the risk (incidence) of a disease over time.
- Best observational design.
- Data usually collected prospectively (some retrospective).

The cohort studies are the best for observational studies as the environmental event can be *assessed* before any disease outcome.

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.



Advantages

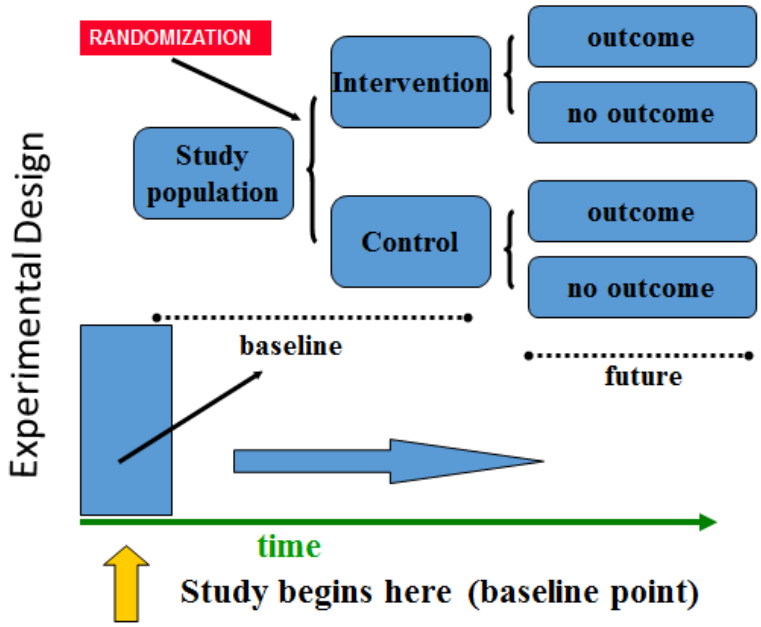
1. Valuable when *exposure* is rare
2. Examines multiple outcomes of a single exposures
3. Temporal relationship is known; Exposure is measured before disease; so no temporal ambiguity (vs. cross-sectional studies)
4. Allow direct measurement of risk
5. Minimize bias in ascertainment of exposure
    - ✓ Exposure status determined before disease detection (avoid information bias); Exposure is measured before disease - so disease cannot influence the amount of error with which exposure status is measured
    - ✓ Subjects selected before disease detection (avoid selection bias); so disease status does not influence of subjects

Limitations

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 consequences of some treatment on some outcome.

Types of experimental studies:

1. **Clinical** trial: on patients in clinical settings.
2. Field trial: on healthy people in the field.
3. Community trial: on the community as a whole.

Individuals are allocated in to treatment and control groups by the investigator.

- ✓ If properly done, experimental studies can produce high quality data.
- ✓ They are the *gold standard* study design.

The quality of "Gold standard" in experimental studies can be achieved through:

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

2. **Blinding**: Denying information on treatment / control status (single "the subject", double "the subject + the researcher" or triple "the subject + the researcher + the data collector" blinding). This helps to avoid observation bias.

3. **Placebo**: an inert material indistinguishable from active treatment. It is used to avoid Placebo effect: tendency to report favourable response regardless of physiological efficacy. (Placebo is used as blinding procedure)

RCT (Randomized Controlled Trial)

An experimental design with subjects randomly assigned to “treatment” and “comparison” groups.

- Clinical trials are the most well-known experimental design.
- The ultimate design in testing causal hypotheses as there is random assignment to groups.
- Provides most convincing evidence of relationship between exposure and effect. So, if RCT results contradicted previous studies results, then its result are considered the true. It is not unexpected to find that observational studies find different results than for clinical trials. For example there have been hundreds of observational studies demonstrating that hormone replacement was protective for women. However, when this was put to a clinical trial, the surprising result was that hormone replacement was not protective

  <u>Experimental and observational studies</u>

  A common goal for a statistical research project is to investigate causality, and in particular to draw a conclusion on the effect of changes in the values of predictors or independent variables on dependent variables or response. There are two major types of causal statistical studies: experimental studies and observational studies. In both types of studies, the effect of differences of an independent variable (or variables) on the behavior of the dependent variable are observed. The difference between the two types lies in how the study is actually conducted. Each can be very effective. An experimental study involves taking measurements of the system under study, manipulating the system, and then taking additional measurements using the same procedure to determine if the manipulation has modified the values of the measurements. In contrast, an observational study does not involve experimental manipulation. Instead,

data are gathered and correlations between predictors and response are investigated.

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.

Sorry for mistakes

Good luck