Medical statistics

Lecture 2: Types of medical studies.

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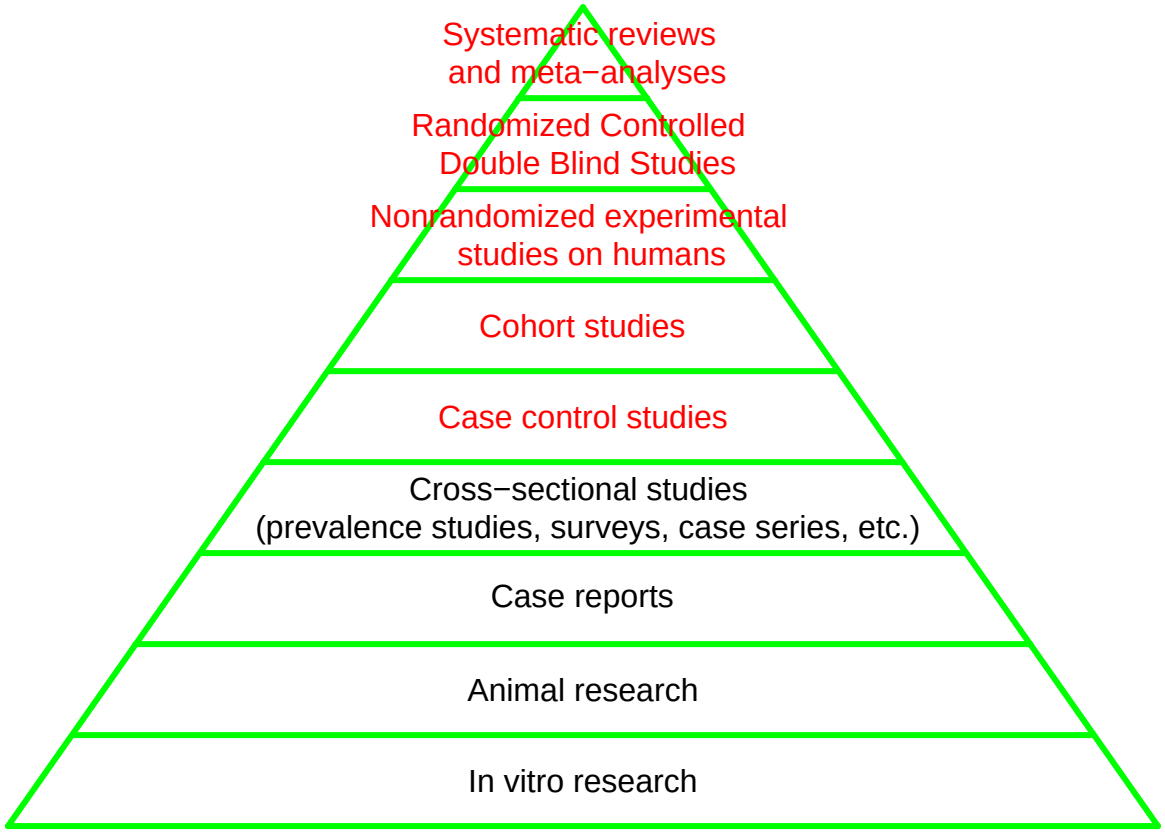
The evidence pyramid – Motivated by: http://library.downstate.edu/ebm/2100.htm

Clinical research,
providing results that are relevant
for the actual clinical practice



Least relevant sources of information
(background information, generating hypotheses for more serious studies, etc.)

Although the lower 3 levels of the pyramid are of great scientific importance, their results do not provide evidence that can directly be used in the clinical practice.

The closer to the top of the pyramid, the more reliable is the study as a source of real clinical evidence.

In the present course we consider only the topmost 6 levels of the pyramid.

Important types of medical research

- Epidemiological (non-interventional) studies
  - Cross-sectional studies
  - Cohort studies
  - Case-control studies
- Experimental research – (randomized) clinical trials, other intervention studies

Some terms concerning epidemiological studies:

Exposure – behavioral, environmental or clinical factor, that could potentially influence incidence in certain diseases or mortality.

smoking, alcohol drinking, low birth weight, antidepressant use, vaccination against diphtheria, working as a high school teacher, living in industrial areas, etc.

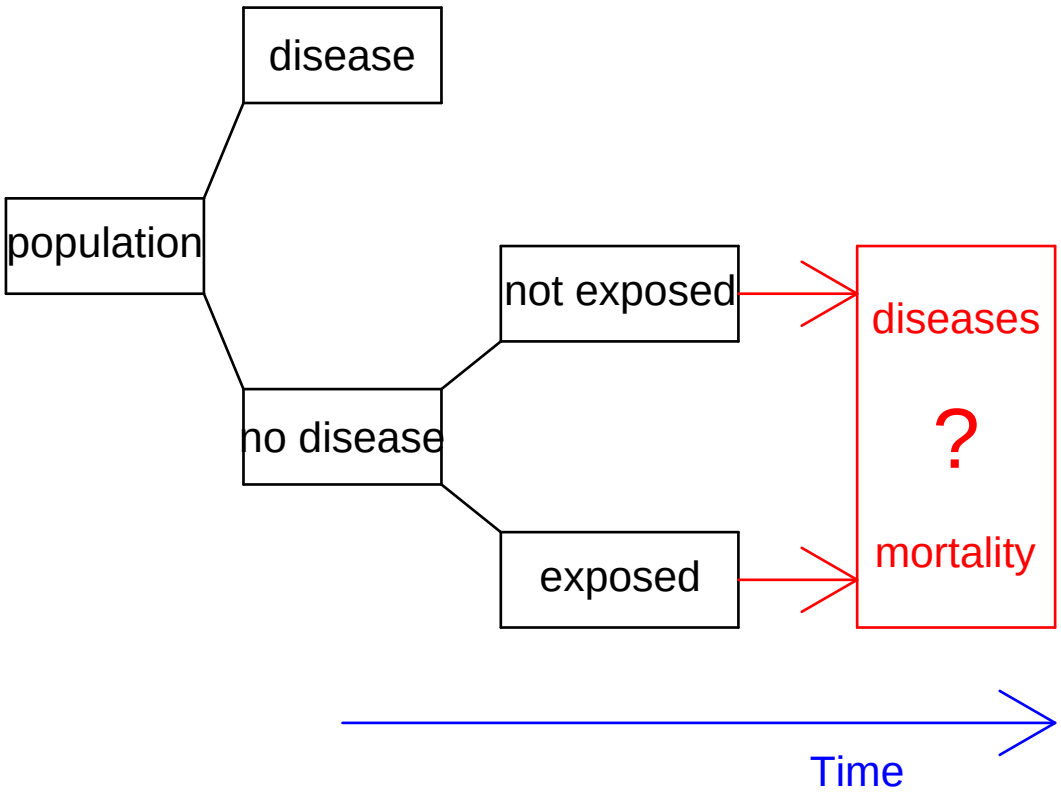
Outcome: disease (cancer, cardiovascular diseases) or death

The main purpose of an epidemiologic study: investigate, whether there is an effect of exposure on outcome.

Cohort studies

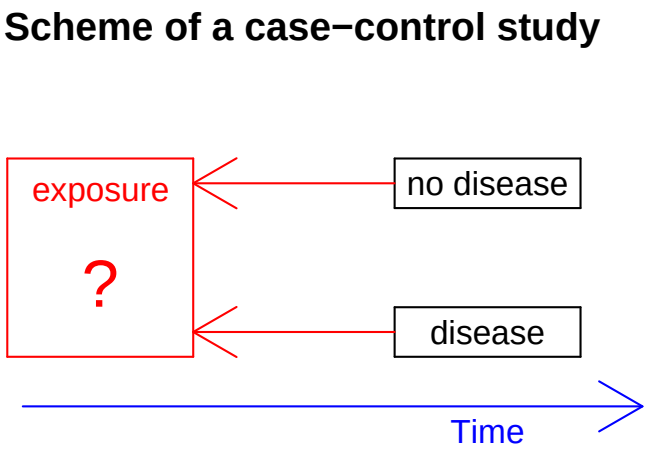
In a cohort study, a large group of individuals, often a well-defined population, is followed over time, to establish the long term effect of initially measured levels of exposure on different health outcomes (diseases, mortality).

Scheme of a cohort study



Case-control studies

A case-control study is a retrospective study that starts with the identification of persons with the disease of interest and a suitable control (or reference) group of individuals without the disease. A relationship of a risk factor to the disease is examined by comparing the diseased and non-diseased with regard to how frequently the risk factor is present.

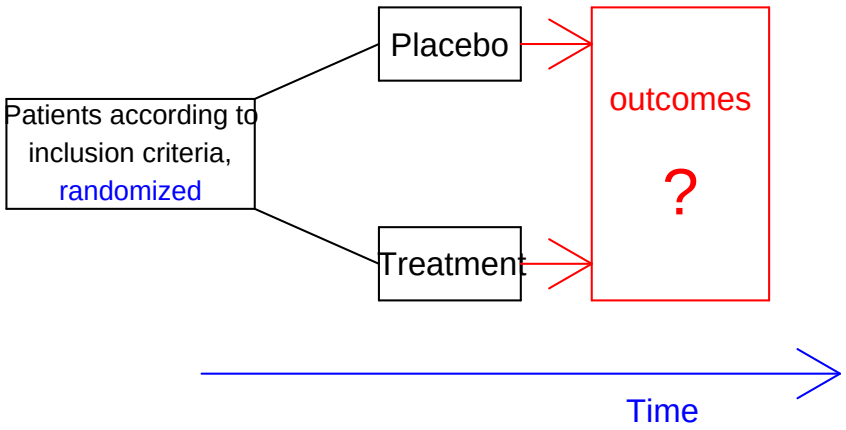


Randomized clinical trials

In this subjects with a disease are randomised to one of two (or more) treatments, one of which may be a control treatment. The importance of randomization is that we know in the long run treatment groups will be balanced in known and unknown prognostic factors. It is important that the treatments are concurrent - that the active and control treatments occur in the same period of time.

In a correctly randomized clinical trial, any systematic differences in outcomes between the two groups to be compared, can only arise due to different exposure (treatment).

Scheme of a clinical trial



Cross-sectional studies

A cross sectional study measures the prevalence of health outcomes or determinants of health, or both, in a population at a point in time or over a short period.

- A survey about health behavior in a certain population.
- Overview of patients with a certain diagnosis (age, sex, clinical data).
- Prevalence studies – to estimate the percentage of people with given disease in the population.

More cross-sectional studies

- Reference ranges for a given quantity (Hgb level, blood pressure)

- Studies of diagnostic tests

More study types

Non-randomized trials: first phases in drug research, to assess the toxicity, optimal dose and possible clinical effect of the new drug.

Dose-response studies

Studies of pharmacokinetics and pharmacodynamics of the drug: bioavailability, biological response to the given dose

Single case studies

to describe a rare case (one of the few study types that does not require statistical analysis)

Another way to classify studies:

Retrospective studies

The study involves collecting data about events that happened before the study was designed (surveys, archive data, etc.) and not about events that will happen in future.

Prospective studies

The study involves data collection about events that are going to happen in the future. Often the resulting data can be analyzed several years after the study has been started.