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Overview of Epidemiological Study Designs

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Overview of Epidemiological study Designs

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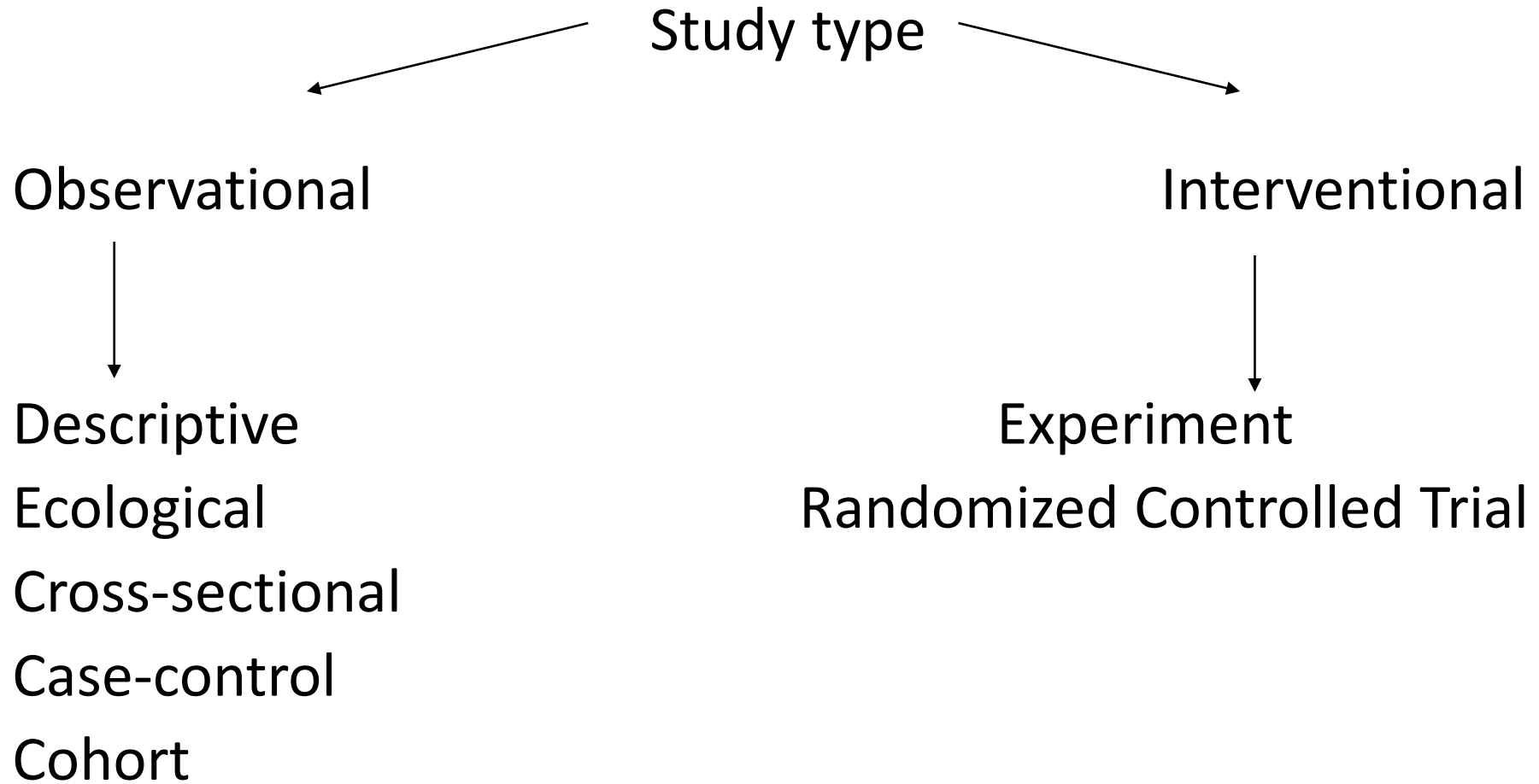
Objectives

- Understand the different epidemiological study types
- Get to know what is involved in each type of study
- Understand the strengths and Limitations of each study type

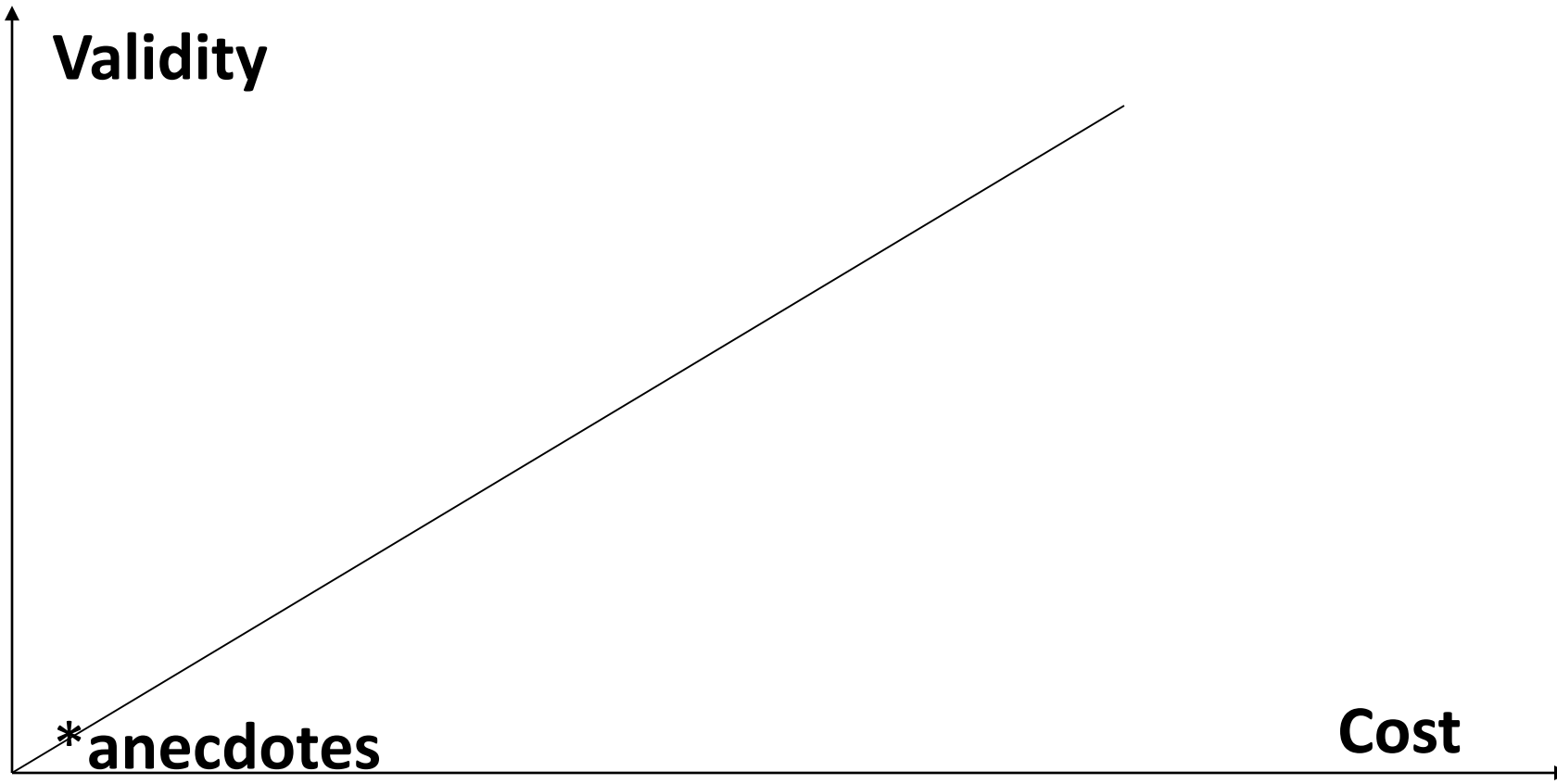
Key terms

- Population
 - Consists of all elements and is the group from which a sample is drawn
- A sample is a subset of a population
- Parameters
 - Summary data from a population
- Statistics
 - Summary data from a sample
- Validity
 - Extent to which a conclusion or statistic is well-founded and likely corresponds accurately to the parameter.

Hierarchy of Evidence



Overview of Epidemiologic Study Designs



Understanding What Physicians Mean:

In my experience ... once.

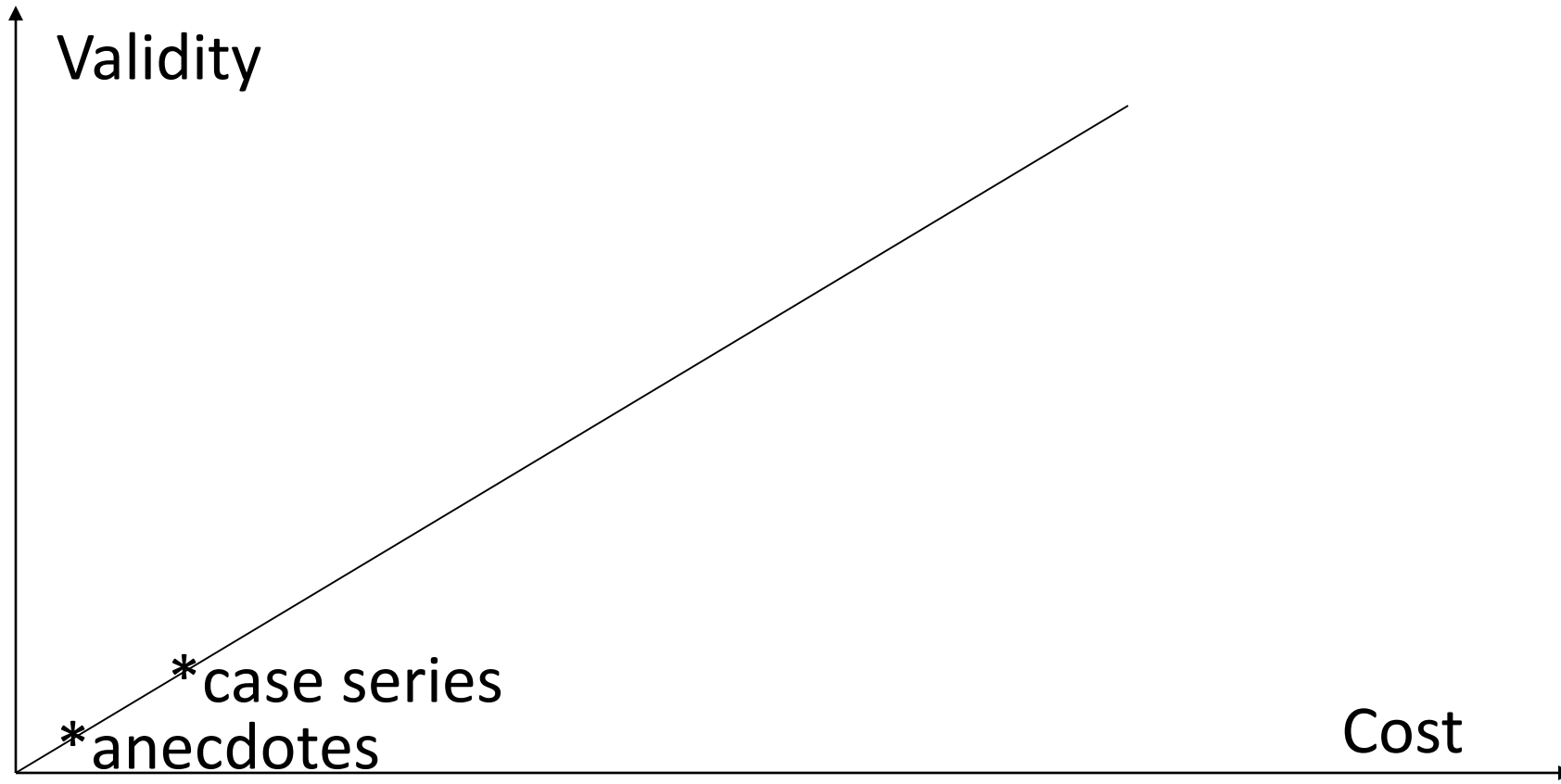
In case after case ... twice.

It is believed that ...I think.

*It is generally believed that ... a couple of other guys think
so too.*

In a series of cases ... thrice.

Epidemiologic Study Designs



Case Series

- Physicians report on a series of cases and make inference about causation.
- Have a denominator of only one or a small number of cases.
- No comparison arm
- Hypothesis development

Case series

- Dr. McBride, an experienced obstetrician in Australia, saw four babies with very rare limb deformities.
- Each of the mothers had taken thalidomide during pregnancy.



Case series

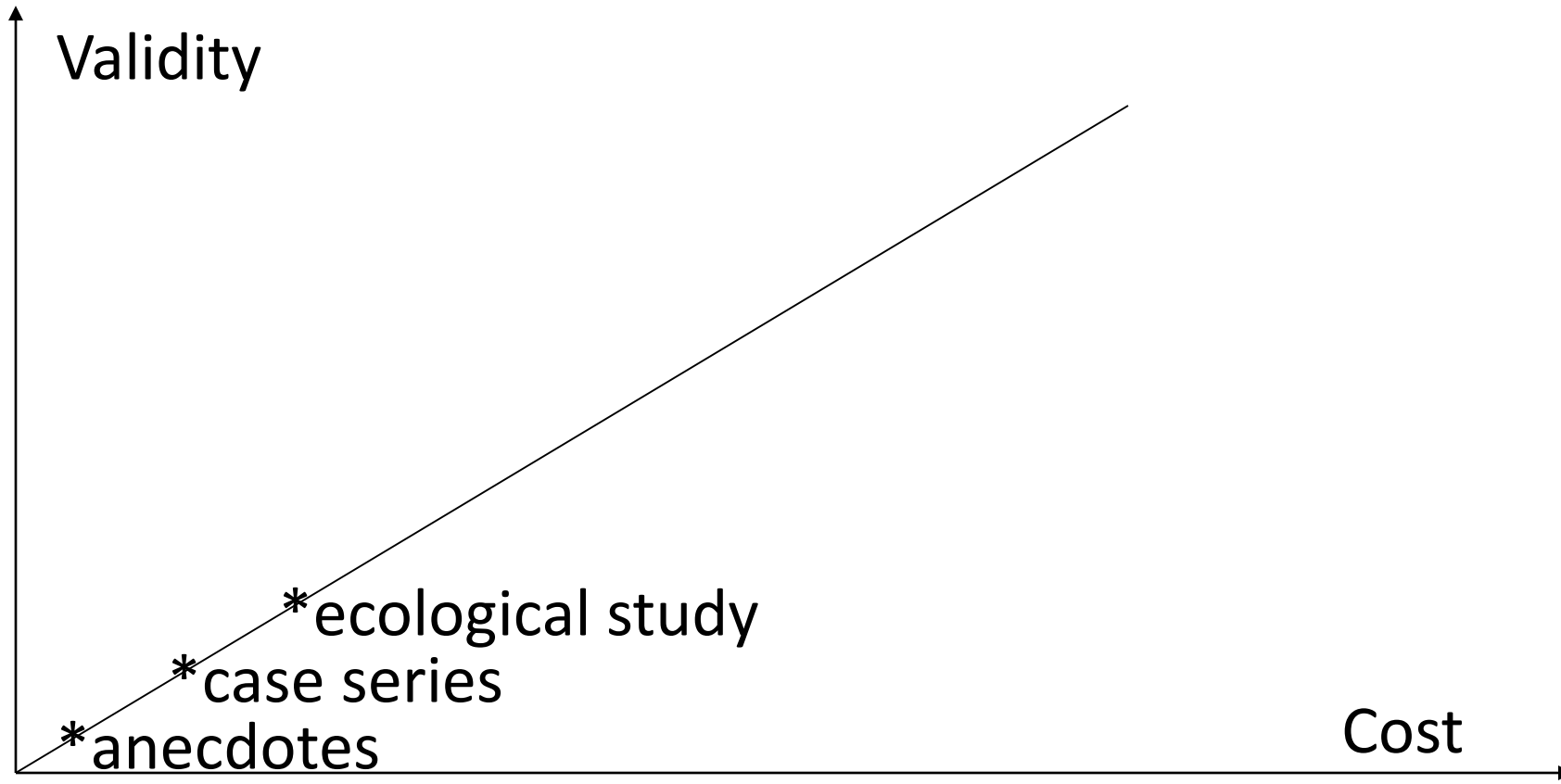


- This case series study worked. Eventually he found 9 cases, and thalidomide had been used in specific weeks of pregnancy
- Case series sometimes work. The disease must normally be very rare, and the relationship with exposure very strong
- Recent case series on dolutegravir and neuro tube defects in Botswana.

Distinguishing study Designs

- In a case series:
 - Data is available on only those with the outcome(The cases)
 - Inference is based on some characteristic (“exposure”) being unusual (usually thought to be rare except in the cases).

Epidemiologic Study Designs

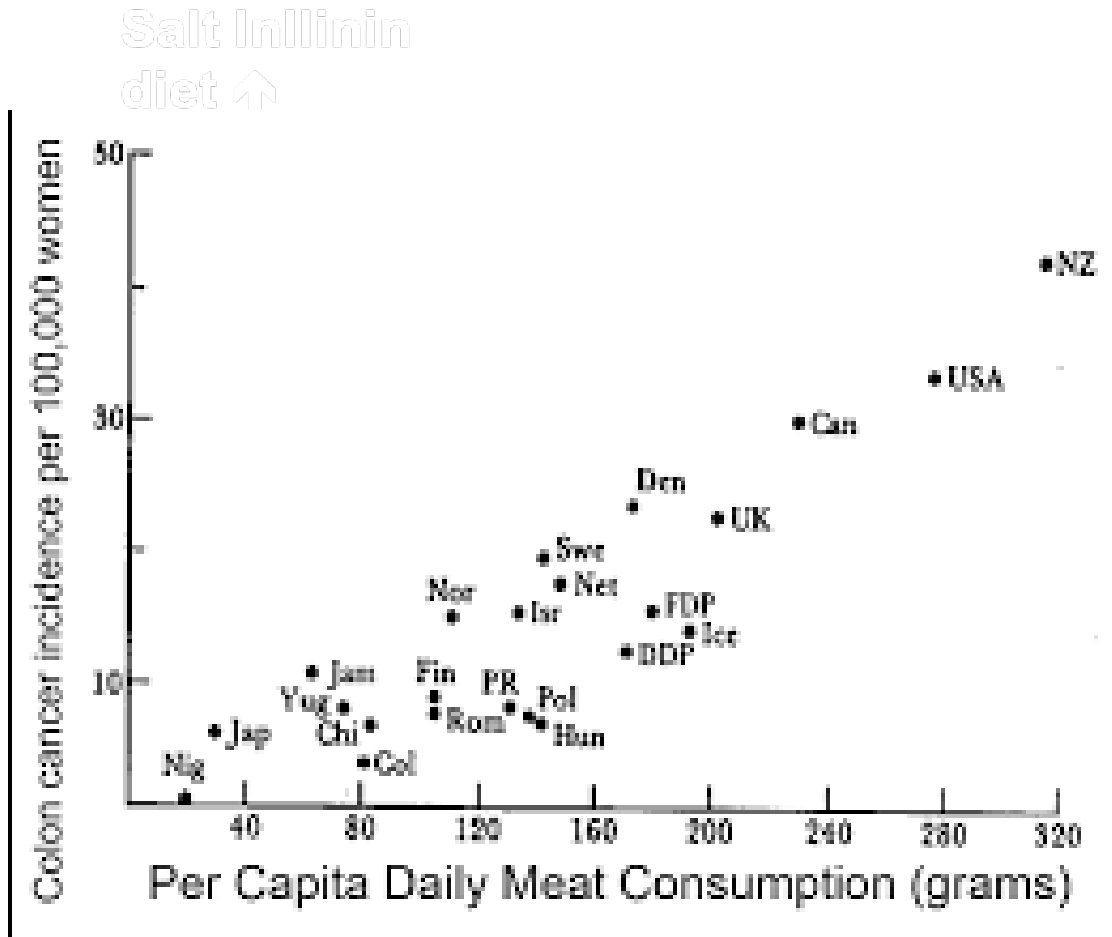


Study designs

- Ecological study design
 - The unit of analysis is the group as opposed to the individual
 - There is no data on exposure status and outcome for each individual in it.
 - Susceptible to the ecological fallacy

Ecological study

- Study in which units of analysis are populations rather than individuals



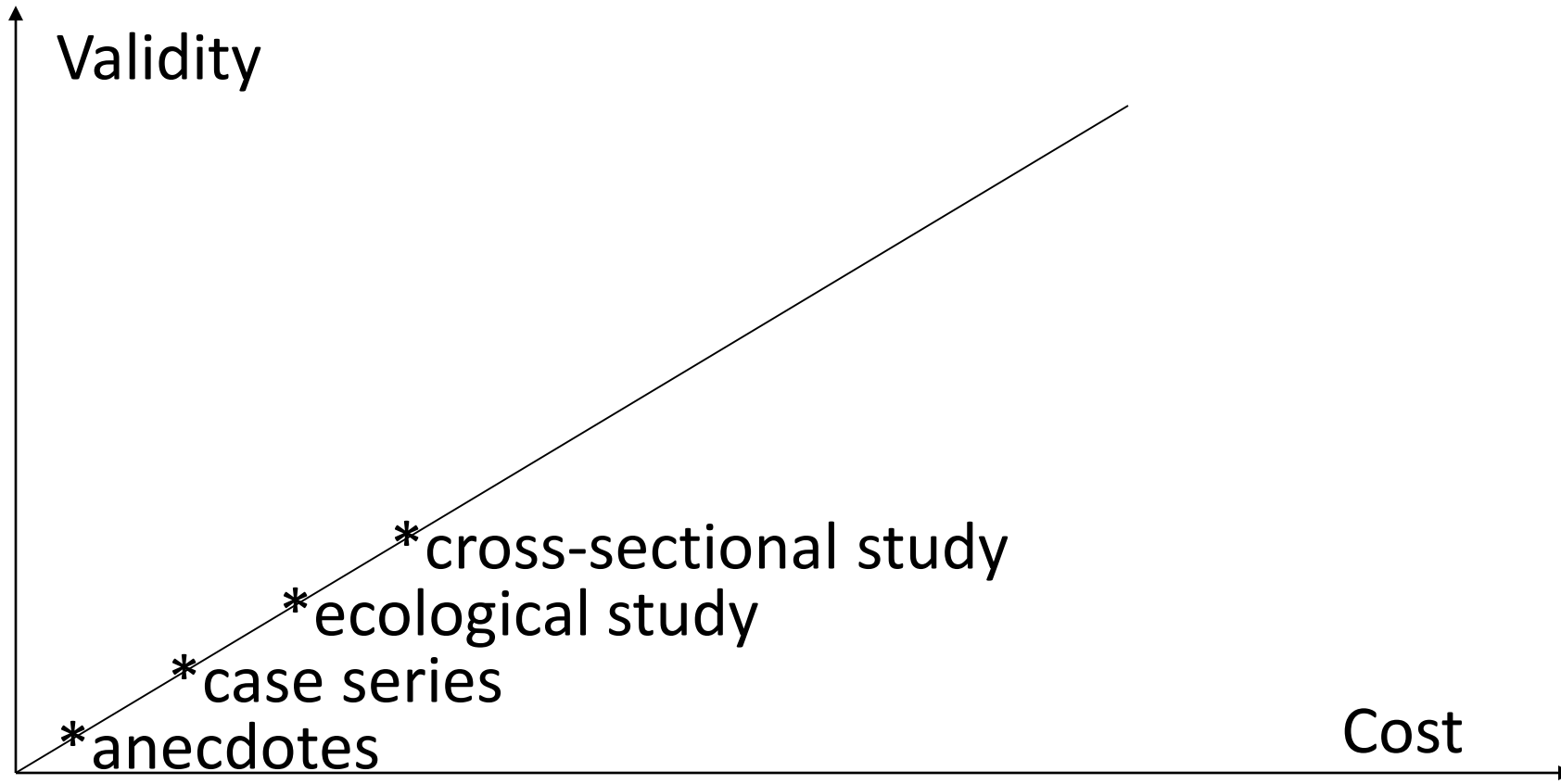
Strengths

- Exposure data often only available at area level.
- Differences in exposure between areas may be bigger than at the individual level, and so are more easily examined.
- Utilization of geographical information systems to examine spatial framework of disease and exposure.

• Limitations

- Measures of exposure are only a proxy based on the average in the population. (ecological fallacy).
- Potential for systematic differences between areas in recording disease frequency. For example there may be differences in disease coding and classification, diagnosis and completeness of reporting between different countries.
- Potential for systematic differences between areas in the measurement of exposures.
- Lack of available data on confounding factors.

Epidemiologic Study Designs



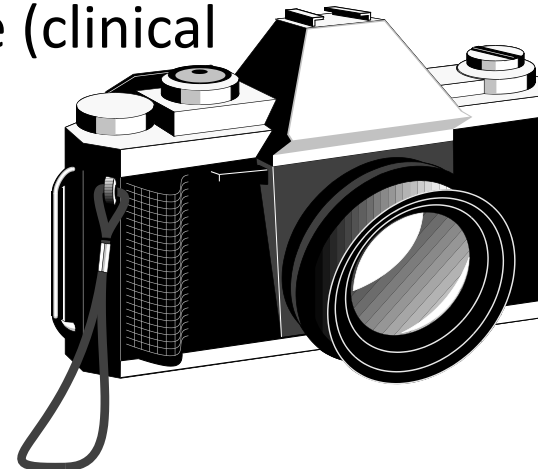
Cross sectional studies

- May also be a prevalence survey
- Exposure and disease status are assessed simultaneously among individuals in a well defined population



Cross-sectional Studies

- Measure the prevalence of disease in a population at a snap shot moment
- Measure the true burden of disease in a population as:
 - they usually obtain more accurate information than routinely collected data
 - they detect cases which have not presented to medical care (clinical iceberg)



- Comparison of exposed group to unexposed group regarding outcome.
- Temporal ordering not clear
- Used to generate relations between exposure and outcomes and to generate hypotheses.
- Length biased sampling: Cases with long duration are over represented.

Chicken or Egg?



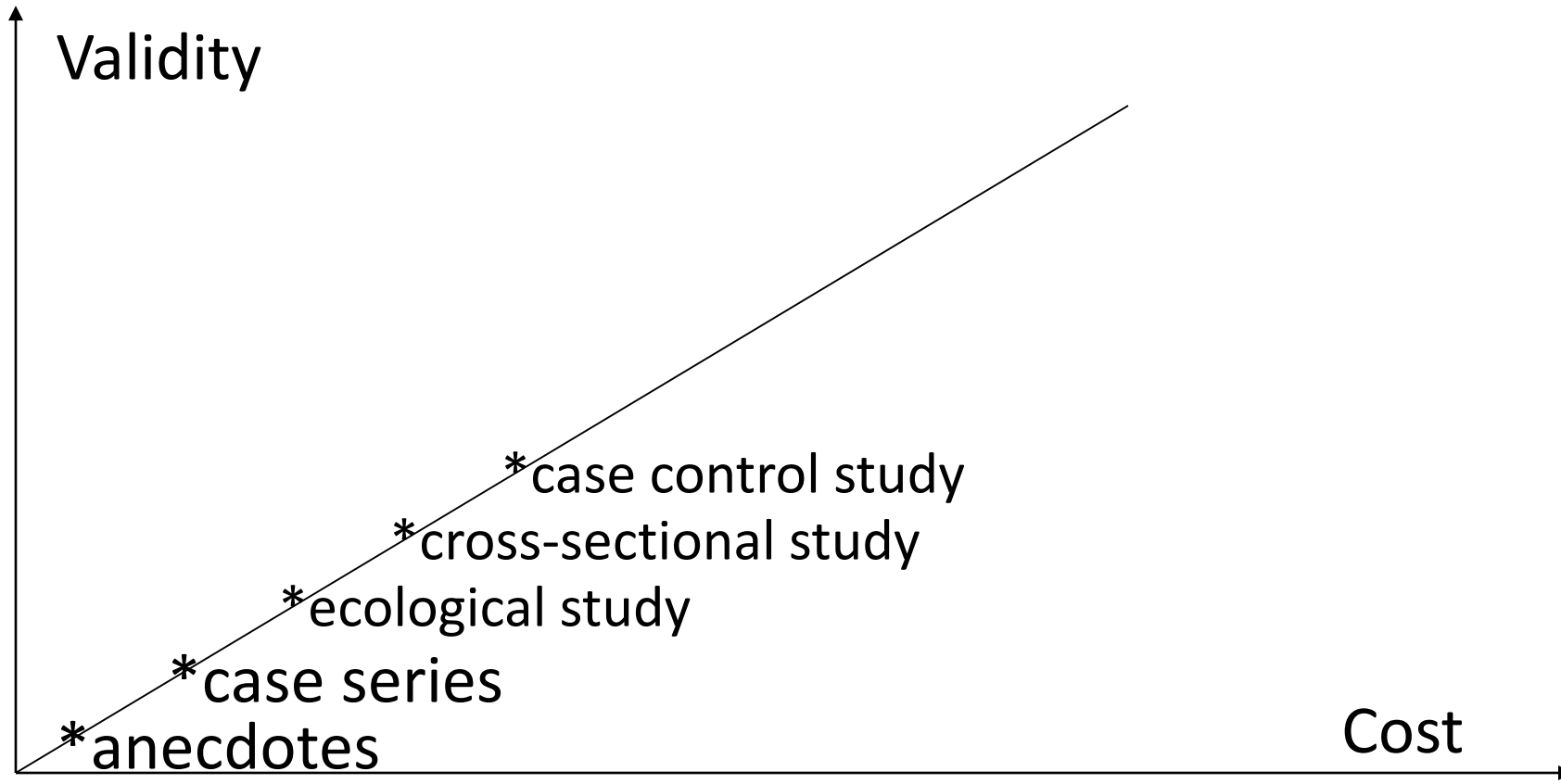
- Strengths

- Used to prove and/or disprove assumptions
- Not costly to perform and does not require a lot of time
- Captures a specific point in time
- Contains multiple variables at the time of the data snapshot
- The data can be used for various types of research
- Many findings and outcomes can be analyzed to create new theories/studies or in-depth research

- Limitations

- Cannot be used to analyze behavior over a period to time
- Does not help determine cause and effect
- The timing of the snapshot is not guaranteed to be representative
- Findings can be flawed or skewed if there is a conflict of interest with the funding source

Epidemiologic Study Designs



STUDY DESIGNS

CASE-CONTROL STUDIES

Study in which you select cases with a disease and compare their past exposures with a selected group of non-cases (controls).

Case-control study

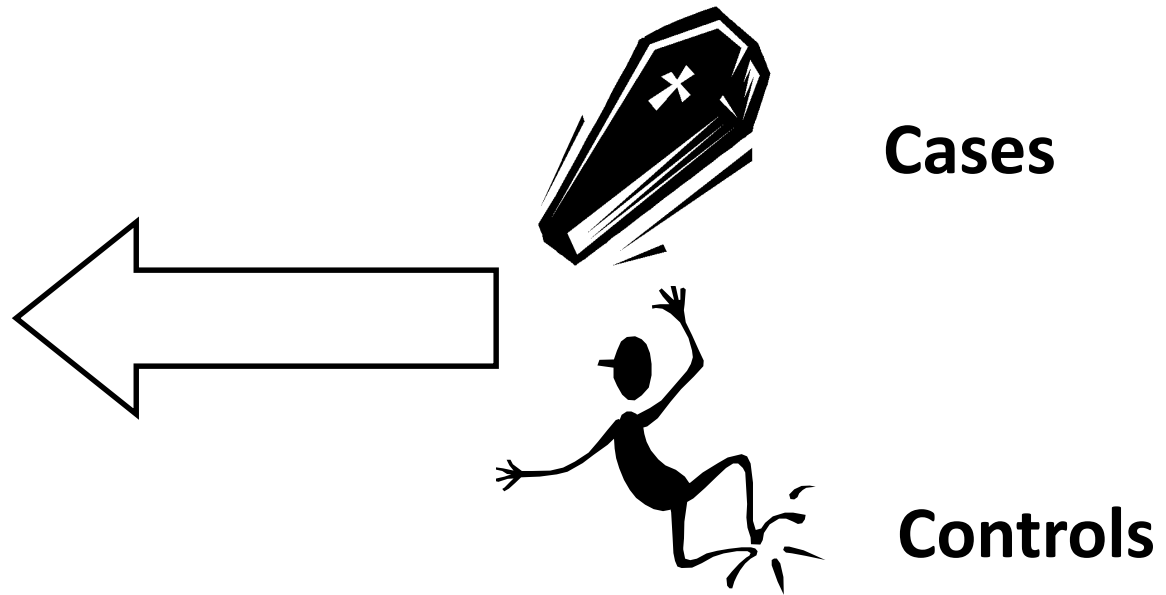
- Type of observational study
- Groups for comparison defined by whether they;
 - Have disease or outcome of interest (a case)
 - Do not have disease or outcome of interest (a control)
- Intuitively obvious and deceptively simple design - extension of clinical history
- Sampling in case control studies
 - Risk set sampling
 - Cumulative based sampling
 - Case cohort sampling

Case-control study

Past exposures



Current outcome



DISTINGUISHING STUDY DESIGNS

CASE-CONTROL STUDIES:

Start by identifying cases with the disease.

Case definition

- Pathological - histological diagnosis of breast cancer
- Radiological - CT scan of a brain tumour
- Microbiological - Sputum positive TB
- Clinical - Parkinson's disease diagnosed by neurologist.
- Self-report - GHQ depression

The controls

- Are not what you might think they are. Unlike the usual use of the term, they are **NOT** a group without exposure.
- Should be representative of the source population in which the cases occurred (in particular with regard to exposure).
- Do not get controls based on exposure status

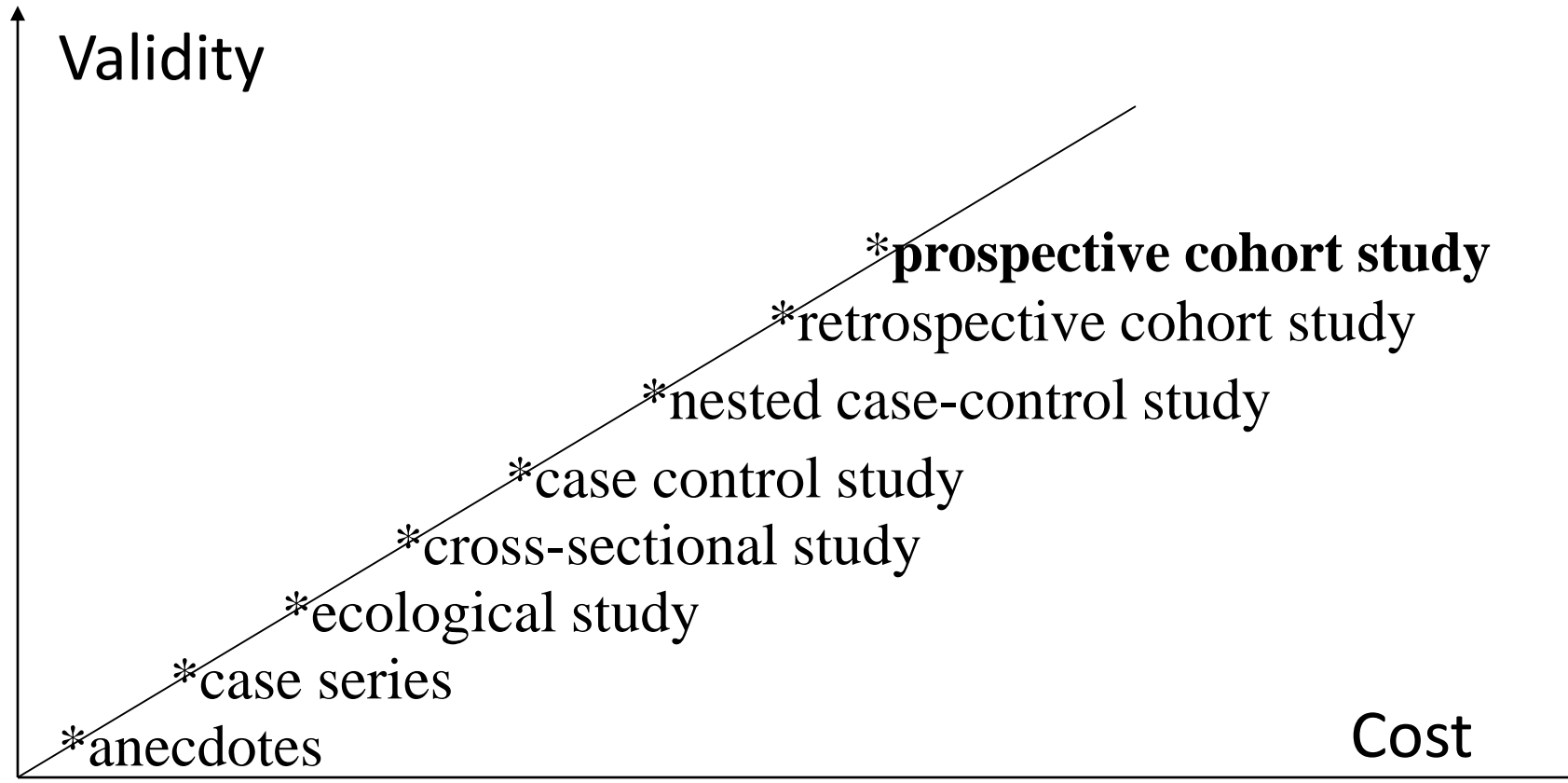
- Strengths

- They are efficient for rare diseases or diseases with a long latency period between exposure and disease manifestation.
- They are less costly and less time-consuming
- Good for dynamic populations in which follow-up is difficult.

- Limitations

- They are subject to selection bias.
- They are inefficient for rare exposures.
- Information on exposure is subject to observation bias.
- They generally do not allow calculation of incidence (absolute risk).

Epidemiologic Study Designs



STUDY DESIGNS

COHORT STUDIES

Study in which you follow a group of people (the cohort) over time to find out how their exposure relates to the occurrence of health outcomes.

DISTINGUISHING STUDY DESIGNS

COHORT STUDIES:

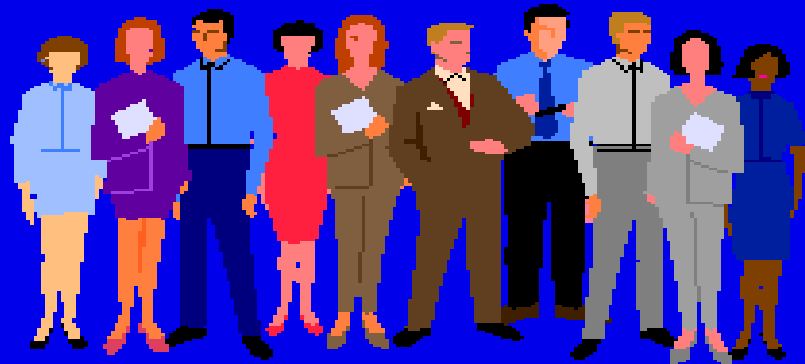
- Start by identifying an “exposed and unexposed” population group before disease occurs.
- Prospective cohort study: you identify the exposed and unexposed population now and follow them for outcomes into the future
- Retrospective (historical) cohort study: Identify a population in the past and find out what has happened to them up to the present with regards to an exposure.

Cohort Study

- Cohort studies are a type of observational study
- For a cohort study
 - Start with a random sample of the target population (just like a cross-sectional study)
 - Establish the exposure status (Exposed or unexposed)
 - Follow-up over time to identify incident cases of disease (outcome)
 - Compare risk of becoming an incident case in those exposed at baseline compared to those who are not exposed

The Cohort Study

Healthy People with
different experiences



time passes

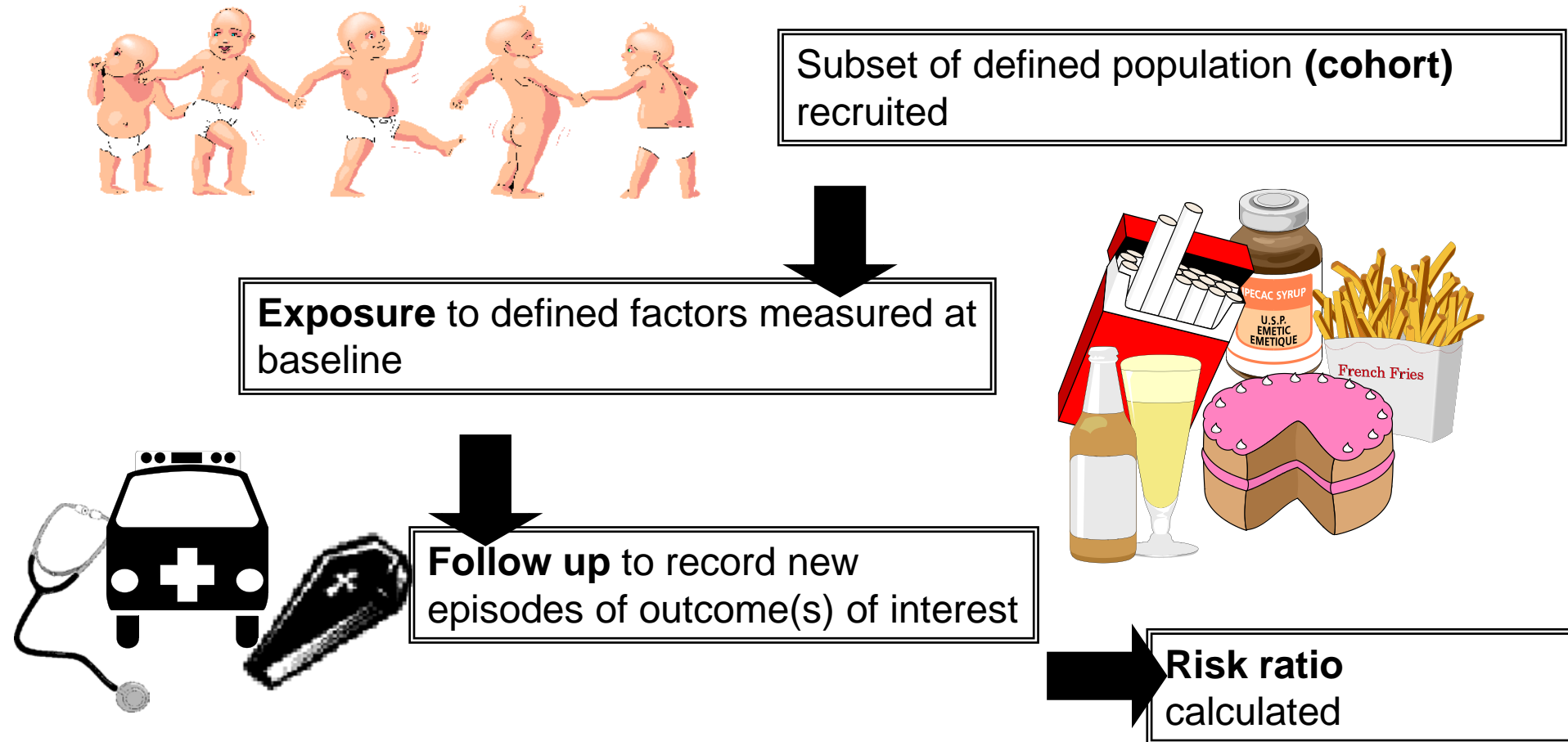


Healthy People
and
Sick People



Study Question:
Who gets sick and who stays healthy?

Main Features of a Cohort Study



- Strengths

- Clarity of temporal sequence
- Allow calculation on incidence
- Facilitate study of rare exposures
- Allow study of multiple effects of a single exposure
- Avoid selection bias at enrolment

- Limitations

- You may have to follow large numbers of subjects for a long time.
- Expensive and time consuming.
- Not good for rare diseases.
- Not good for diseases with a long latency.
- Differential loss to follow up can introduce bias.

DISTINGUISHING STUDY DESIGNS

ECOLOGIC STUDIES

No individual link between exposure and outcome. Unit of measure is the group

CROSS-SECTIONAL STUDIES:

Identify exposure and disease in a population at the same time.

CASE-CONTROL STUDIES:

Start by identifying cases with the disease, get controls and assess for past exposure.

COHORT STUDIES:

Start by identifying an “exposed” population group before disease occurs.

Sources of Exposure and Outcome Data

Exposure data

- Medical records
- Employment records
- Interviews
- Questionnaires
- Medical tests
- Environmental tests

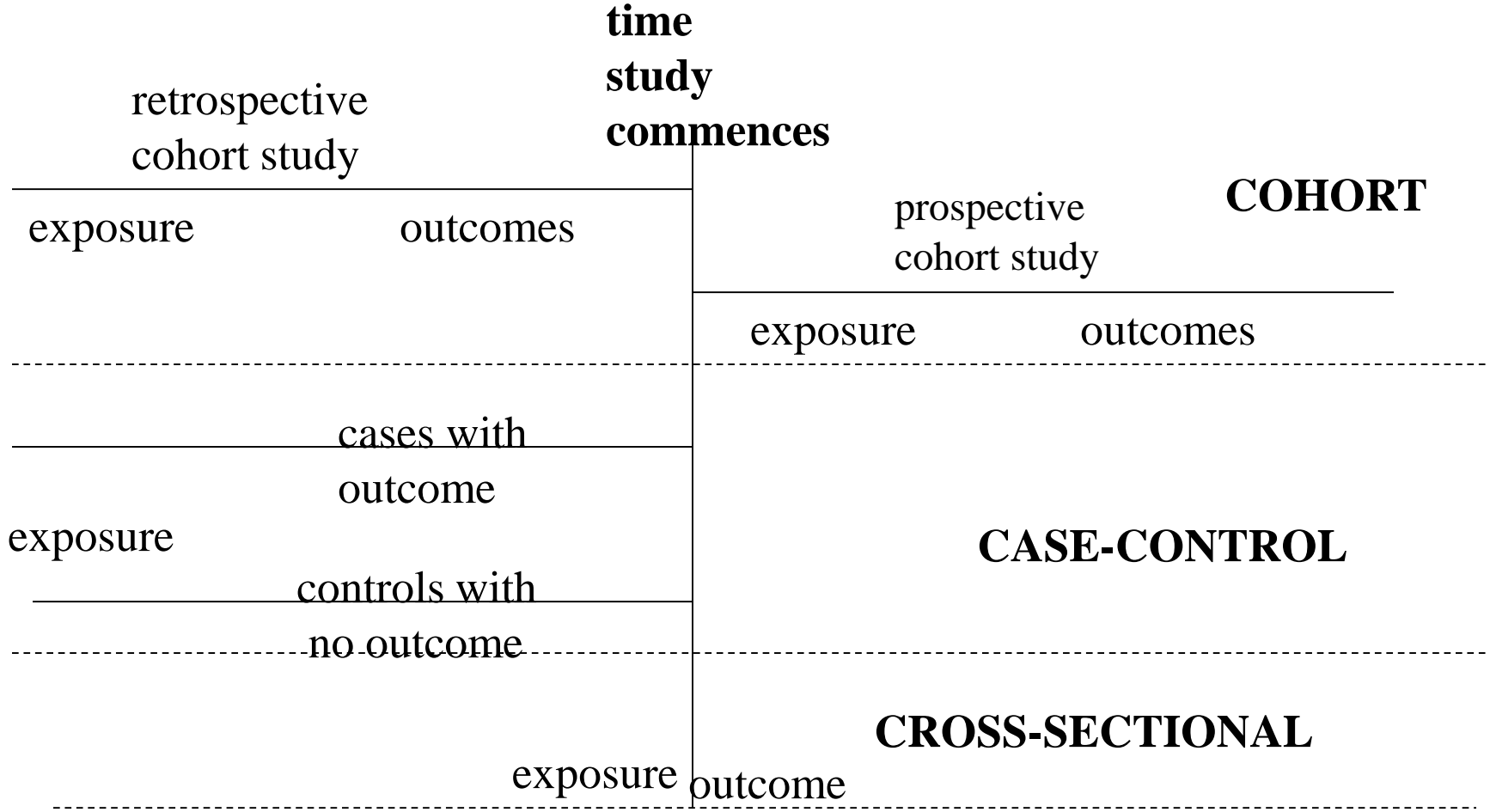
etc.

Outcome data

- Medical records
- Death certificates
- Physical examination
- Questionnaires

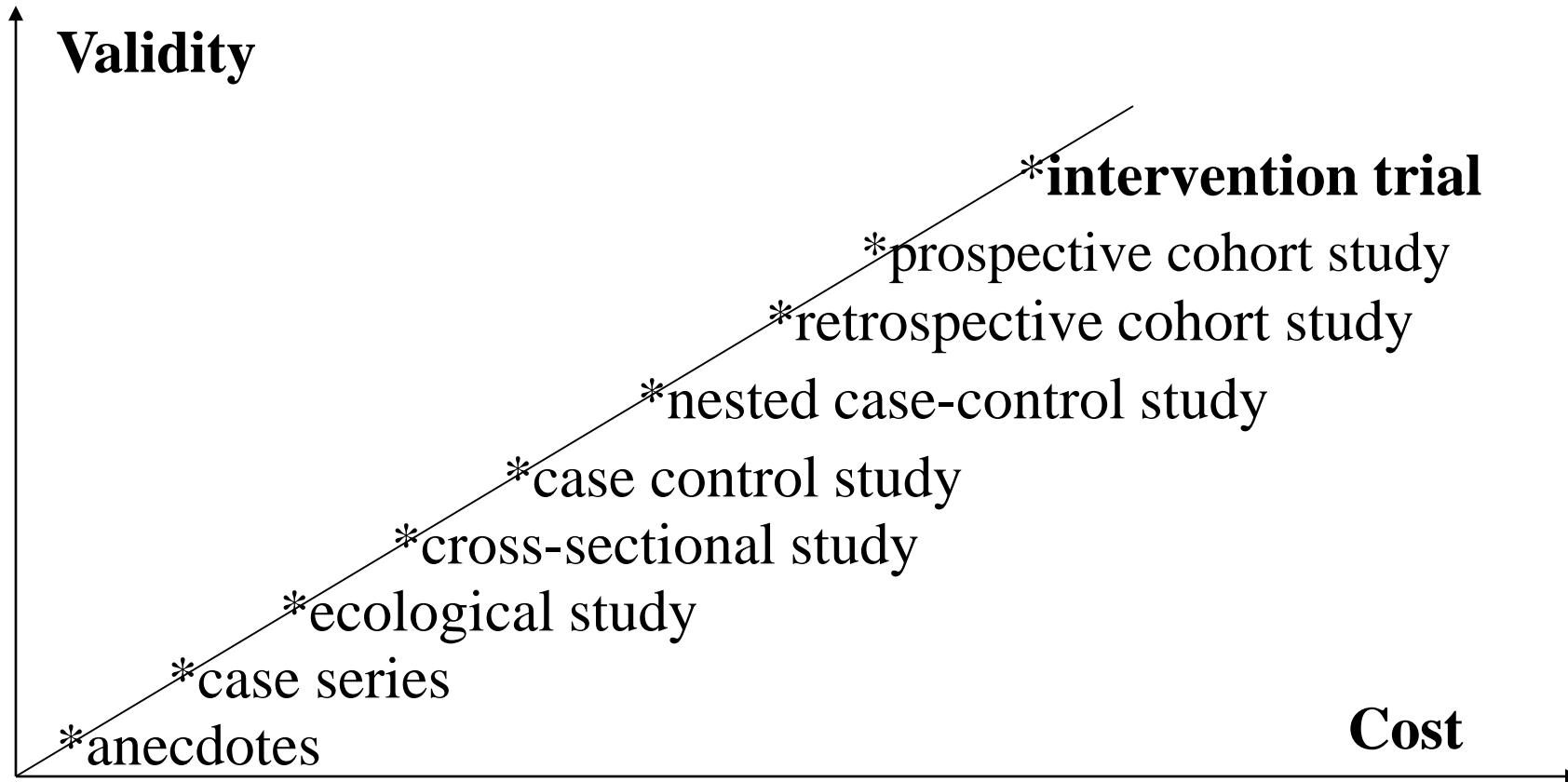
etc.

The 3C Study Types



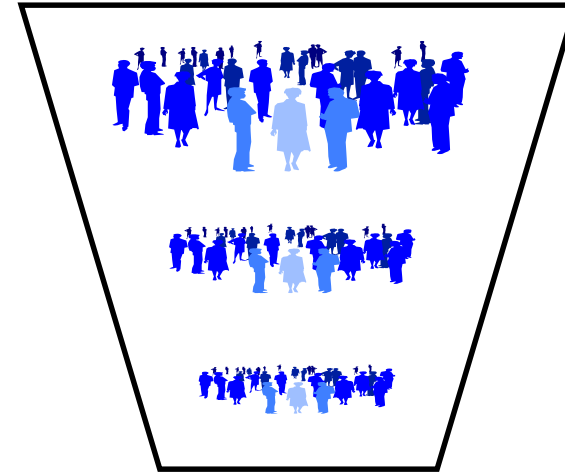
Note: in each of the 3C designs, individuals are either exposed or not exposed. The use of individual data contrasts with ecological studies which are based on group data, usually dependent on geographic location.

Epidemiologic Study Designs



**Doing a
randomised
controlled
trial**

Population
↓
Eligible individuals
↓
Consenting individuals



 **Randomise** 

Intervention

Control group

(Baseline measurements)

Measure & compare outcomes

Look at :
Non-compliance
Completeness of
follow-up

- Strengths
 - Avoid bias related to confounding factors (through a control group)
 - Selection bias (through randomization)
 - Interpretation bias (through double blinding).
- Weaknesses
 - Recruited patients in clinical trials and study experimental conditions markedly differ from the situation in real life.
 - Trials recruit a mix of good and poor responders, so that the average therapeutic response is most often mitigated.
 - Ethics for performing some studies

Key issues in Randomized trails

- Uncertainty principle and clinical equipoise
- Randomization and confounder overlap
- Allocation concealment before trial
- Blinding after trial starts
- Ethical Issues in an interventional study
- Study compliance and loss to follow up.



Why do doctors need to be able to critically appraise RCTs

- They are
 - Gold standard evidence for causation
 - Gold standard assessment of effectiveness of treatment
- BUT:
 - poorly conducted RCT may be biased and poorer evidence than observational study
- CANNOT assume just because a study is described as an RCT that the findings are unbiased

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Thank You

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