Study Designs in Epidemiology

Descriptive and Cross-Sectional Designs

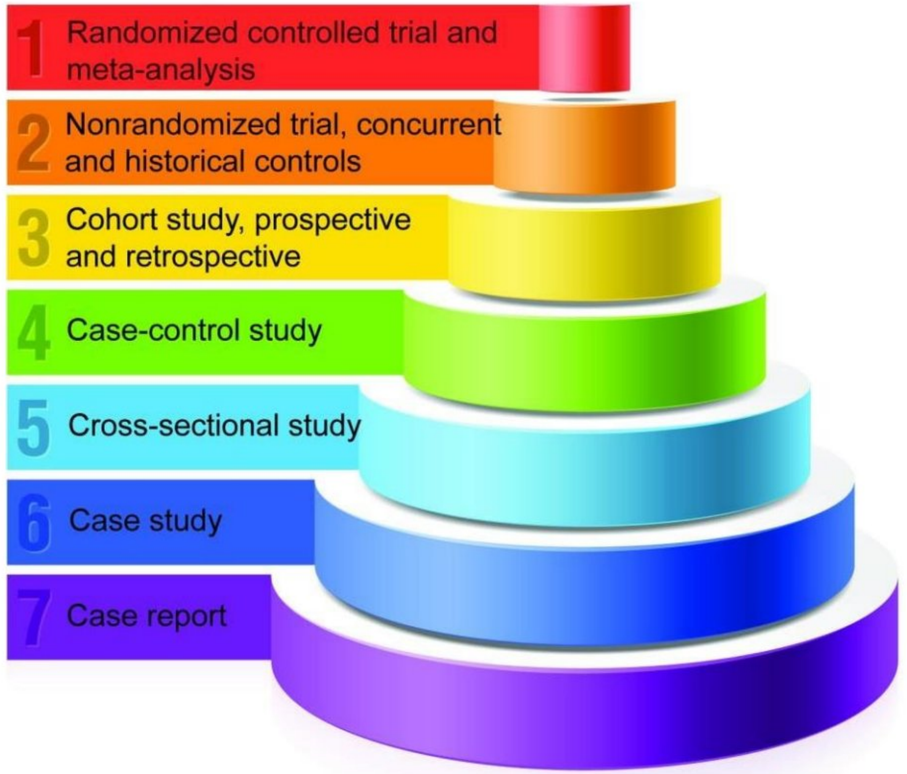

Figure. Hierarchy of Research Design

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Epidemiologic Study Design

Study design is the arrangement of conditions for the collection and analysis of data to provide the most accurate answer to a question (that has been set previously) in the most economical way (that is with the least possible cost).

Types of Epidemiologic Study Designs

I. Based on objective/focus/research question:

1. Descriptive studies → They describe a health problem in terms of what the problem is who's affected, when and where.

Describe: what, who, when, where

2. Analytic studies → They analyze the problem by answering two more question, by answering how does the disease happen, and why it happens

Analyze: How and why

Types of Epidemiological Study Designs

II. Based on the role of the investigator

1. Observational studies

The researcher only investigate the health phenomena that is naturally happening (disease is happening by itself).

- **The investigator observes what naturally happens**
- **No intervention**

2. Intervention/Experimental studies

- **Investigator intervenes: changes things and introduce exposure** to participants, to look for the effect.
- **Researcher has a control over the situation**

Types of Epidemiological Study Designs

III. Based on timing :

1. One-time (one-spot) studies

- **Conducted at a point in time**, we collect all the data needed, **there is no follow up**.
- **An individual is observed at once** and data collected at one point in time.

2. Longitudinal (Follow-up) studies

- **Conducted over a period of time**
- **Individuals are followed over a period of time** to look for certain side effects or changes that are happening to them after a certain exposure.

Types of Epidemiological Study Designs

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

1. Prospective

Data collection occurs forward in time: into the future

2. Retrospective (Previous or historical exposures)

Conducted backward in time: past events

Types of Epidemiological Study Designs

V. Based on type of data they generate:

1. Qualitative studies:

- **Generate textual data**

  There are no numbers, only texts (words) that will be analysed in a special way.

- **Also called exploratory studies**

  Qualitative studies are used in a health field or health problem that we know nothing about, we don't know about how it occurs, why it occurs, we need to know more so we ask people and take their experience and how they feel and what happened to them in a text way, we explore a new field; that is why it's exploratory.

2. Quantitative studies:

Most of the research that we do in health care is quantitative.

- **Generate numerical data**

  The data generated is all numbers, on which we do statistical analysis.

- **Also called explanatory studies**

  They explain more about a health problem.

Types of Epidemiological Study Designs

The most widely used classification:

- **Descriptive studies (who, when, where)**
  describe occurrence of outcome (health problem or disease)

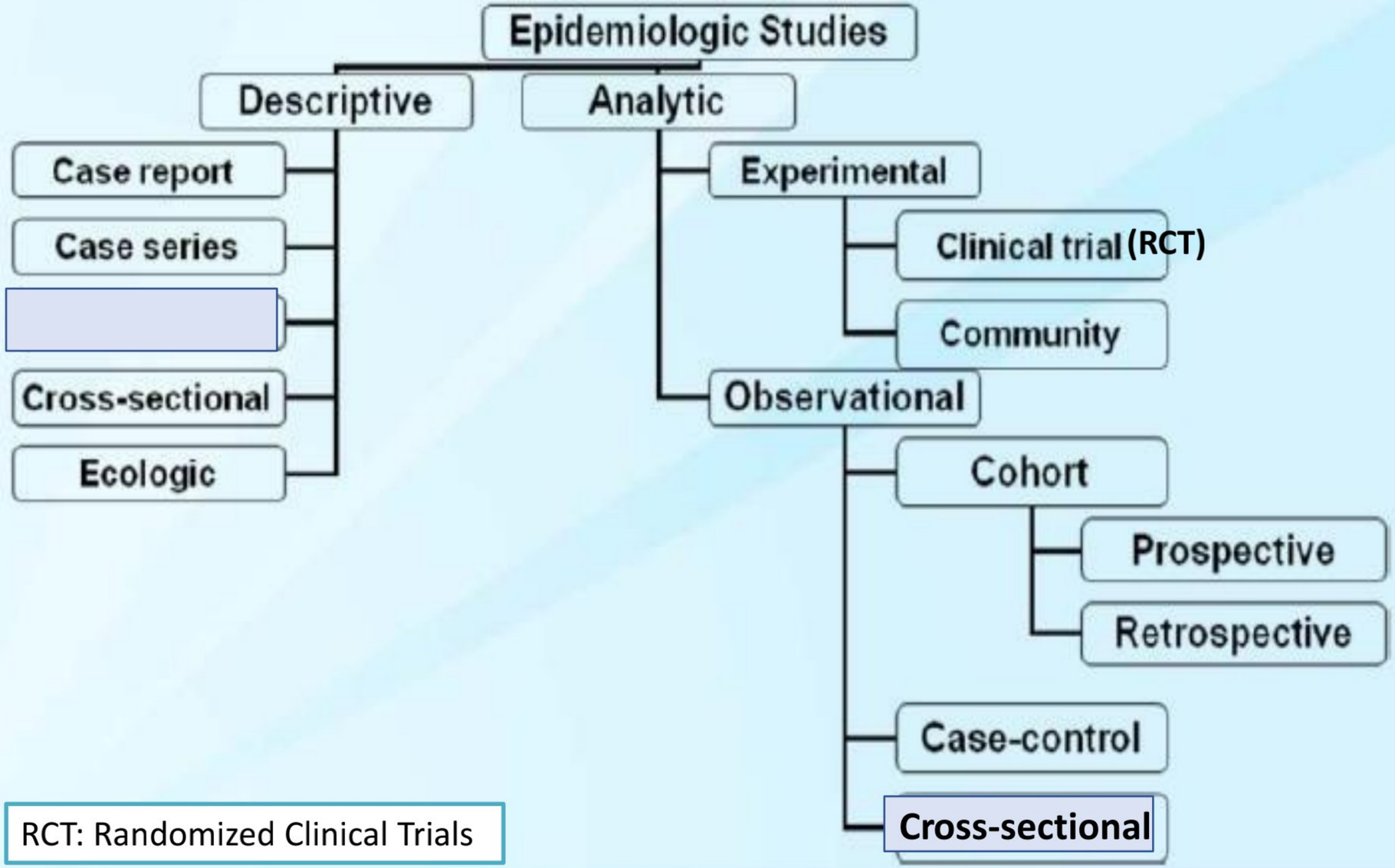
- **Analytic studies (how, why)**
  describe association between exposure and outcome

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

Basic Research 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.

Taxonomy of Epidemiologic Studies



RCT: Randomized Clinical Trials

Descriptive Studies

- Descriptive studies are usually the first phase of an epidemiological investigation. → Usually the starting point.
- These studies are concerned with observing the distribution of disease or health – related characteristics in human populations.

Distribution across person (who), place (where) and time (when).

- Such studies basically ask the questions of what, who, where, and when.
- Useful for generating new hypothesis (provides clues to disease etiology)

→ That if we want to investigate more and explore associations between health problems and risk factors we need to answer why does it happen and how does it happen.

Research Hypothesis

A hypothesis is a supposition (افتراض), arrived at from observation or reflection.

As we said, descriptive studies are helpful for generating new hypothesis from what we've observed.

- **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 (Risk factor) being considered.**
3. **Expected outcome – disease (Results from the risk factor).**
4. **Time response relationship (expectation).**

   → **Does the disease increase with time? Or decrease with time?**

5. **Be understandable, measurable and testable.**

   → **Also, it should be realistic that can be applied in research.**

Develop a research question & Hypothesis

- **General concern – Hb of mother and Birth weight of baby.**

Some investigator have observed that there is some connection between the level of hemoglobin in mother if they had low hemoglobin level or anemia and that led to differences in birth weight of babies or maybe low birth weight

RQ - (Research Question)

- **Is Anemia in pregnancy associated with low birth weight in newborn?**

Null Hypothesis

- **There is no difference in the incidence of LBWs in the mothers who are anemic and those who are not anemic.**

Research Hypothesis

- **The incidence of LBWs in mothers who are anemic is higher than those who are not anemic**

Descriptive studies

This is the most quick and easy and very simple to do

1. Case Reports:

- presentation of a single case or handful of cases
- Generally report a new or unique finding
  - e.g. previous undescribed disease that has been identified recently in a very low number of patients (1 to 5 handful which means 1 to 5 cases)
  - e.g. unexpected link between diseases
  - e.g. unexpected new therapeutic effect
  - e.g. adverse events

- ✓ Things that have not been reported previously in the scientific body , we didn't know this before in books or in research
- ✓ Now it has been newly identified in a very low number and it's worth to be published so that people start to know about it

Descriptive studies

2. Case Series

Experience of a group of patients with a similar diagnosis (Larger than 5 cases)

- **Cases may be identified from a single or multiple sources** maybe physicians in different centers or hospitals have noticed similar cases but still for something new
- **Generally report on new/unique condition**
- **May be the only realistic design for rare disorders**

There are some disorders that one or two or very rare cases may be diagnosed in a whole year, they can be reported as case report or case series and published in scientific journals.

Case reports are in many ways “sentinel events” which can lead to testable hypotheses

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

Case report and Case Series

- **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 in a population unless we do bigger studies

3. Ecological Studies (correlation study)

The ecologic study is a hypothesis generating study. Usually using group-level data (population-level) for example death rate for a total population due to cardiovascular disease or lung cancer in Jordanian population, it examines if two factors are correlated with each other.

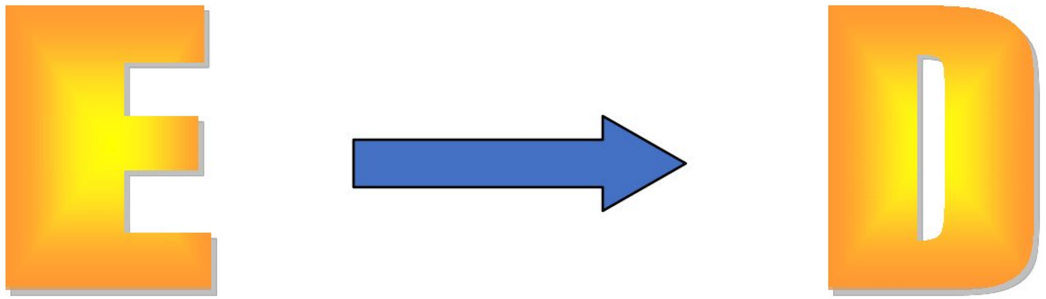
- It involves the collection of events over a defined population base and by the use of denominator data to determine rates.

It results in Ecological Fallacy: Failure in reasoning that arises when an inference is made about an individual based on aggregate data for a group (e.g. [1] Higher rates of coronary heart disease in countries with higher income -> The ecological fallacy is to conclude that people with high income are at high risk for coronary heart disease , this is the fallacy (error) in reasoning or judgment that we could do, **We should not come to conclusion on individual basis of cause & effect relationship between risk factor & disease.**

[2] Higher rates of leukemia in larger cities, [3] higher rates of car accidents in countries or regions with higher smoking rates).

Analytical Epidemiology

Are exposure and disease linked?



Exposure **Disease**

If you noticed taxonomy that we studied before, there's cross-sectional studies in both Descriptive and Analytical, but we will consider it as Analytical in this lecture.

Analytical Studies (testing hypothesis)

- ✓ Testing hypothesis means to make sure whether the hypothesis that has been generated by descriptive studies is right or wrong
- ✓ And we can conclude from analytical study whether the hypothesis is true or false

Observational Studies

- **Cross-sectional**
- **Case-control**
- **Cohort**

The investigator just observes what's naturally happening
We don't intervene in ANYWAY!

Experimental Studies

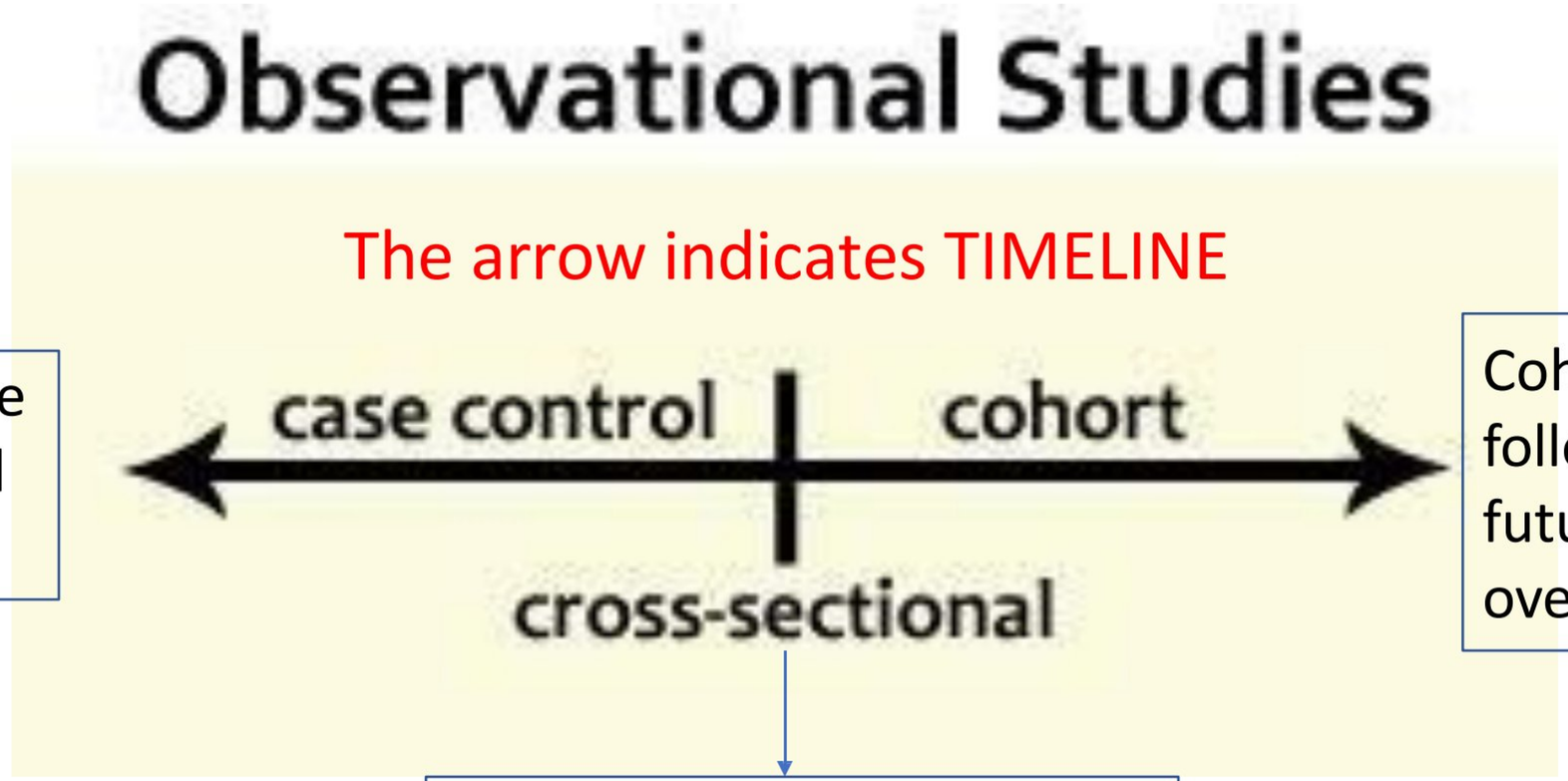
- **Randomized controlled clinical trials** the strongest and best design in experimental and in all studies
- **Community trials**

Observational Studies

Non-experimental study designs:

- Observational because there is no individual intervention
- Treatment and exposures occur naturally
- Individuals can be observed prospectively, retrospectively, or currently

The simplest and quickest to do is a cross-sectional (survey)



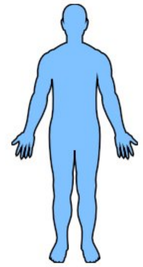
Case control : we collect historical previous data

Cohort : we do follow up into the future to collect data over time

Investigation at this point of time so it's concurrent

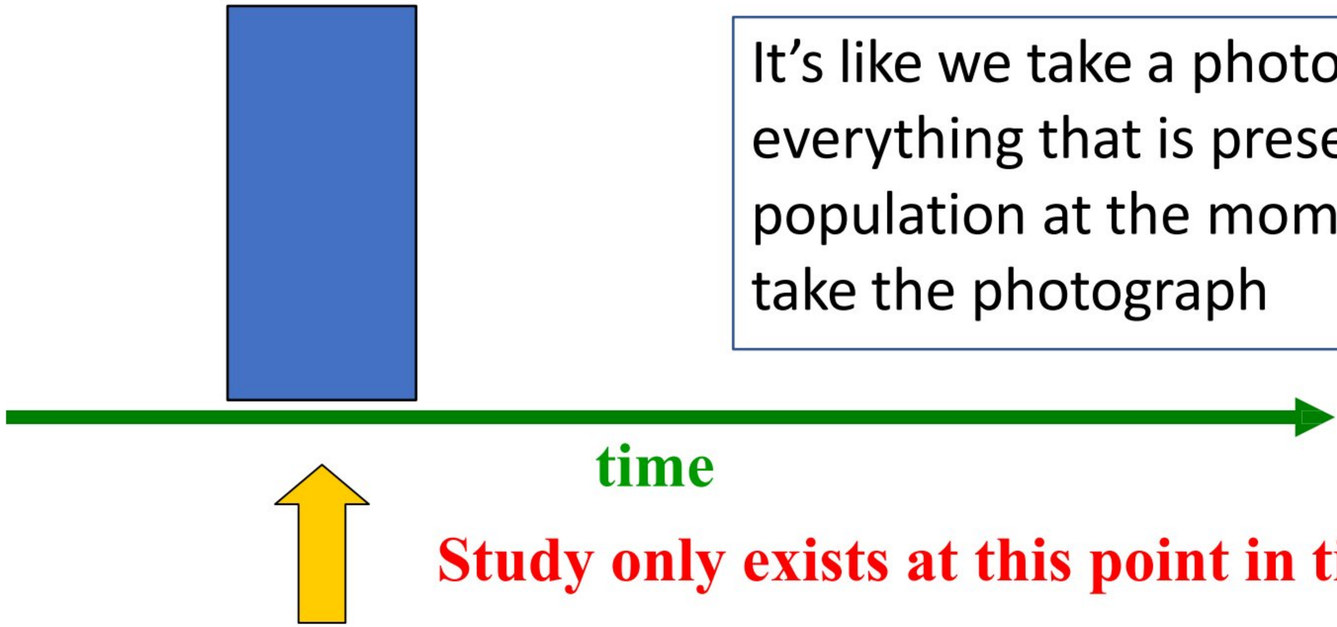
http://www.medbullets.com/step1-stats/1001/types-of-studies

Cross-sectional studies



An “observational” design that surveys exposures and disease status at a single point in time (a cross-section of the population)

We can also describe it as a snapshot of the population



It’s like we take a photograph for everything that is present in this population at the moment when we take the photograph

Cross section al studies are some of the first studies completed because of ease and low cost

Cross-sectional studies

- Based on a single examination of a cross section of population at one point in time, by studying a sample that represents the population.
- Results of which can be generalized to the whole population (provided the sampling has been done correctly). The more the sample represents the target population the more the results will be generalized to the population
- Longitudinal studies are Based on multiple observations in the same population over a multiple points of time.

e.g. What is the prevalence of diabetes in Jordan? We take a representative sample of Jordanian population, and calculate prevalence of diabetes

What is the prevalence of malnutrition among children in Jordan?

A survey of asthma among animal handlers

A survey of dietary habits among university students

Cross-sectional studies

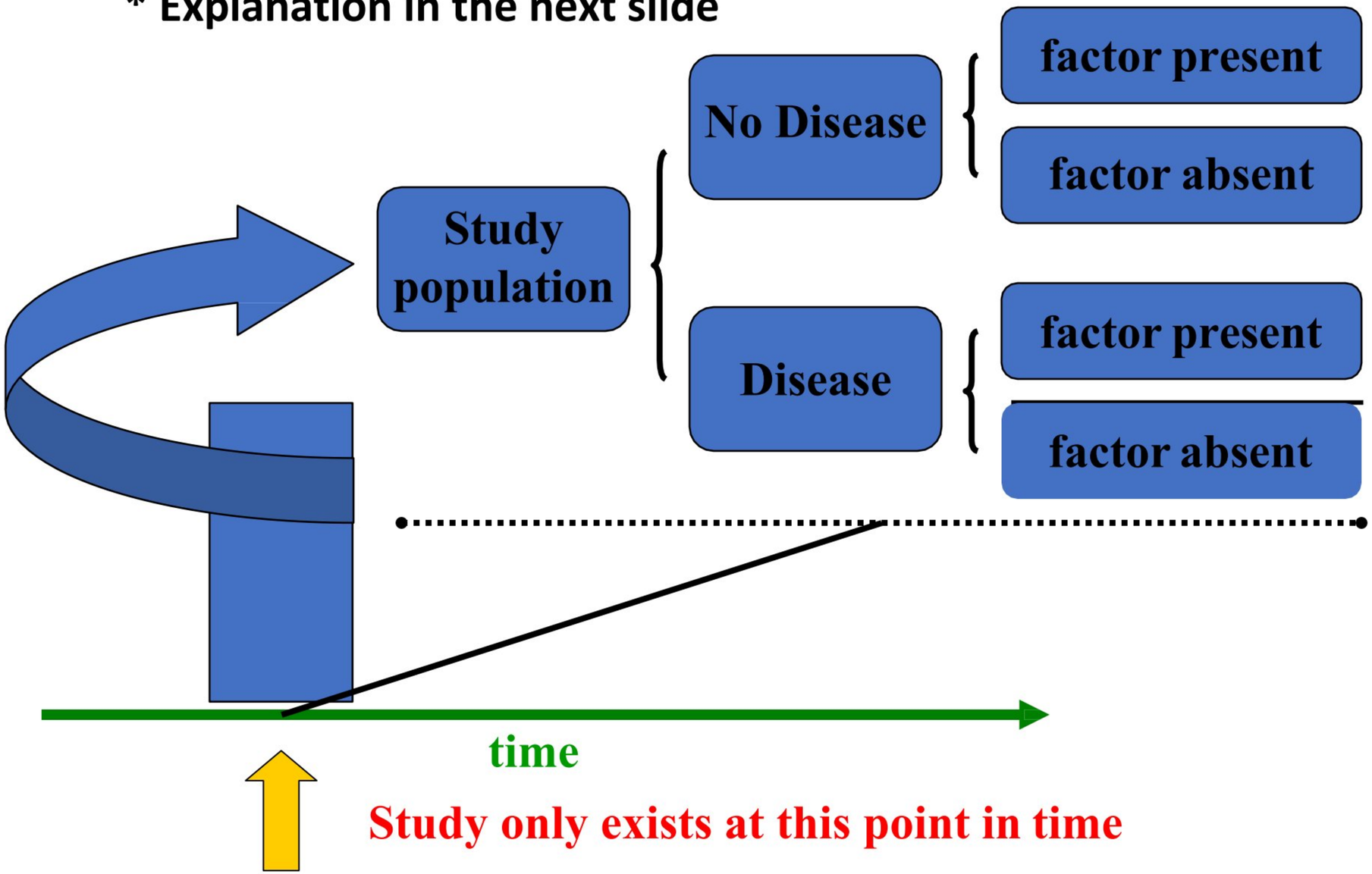
Used to learn more about the disease to explore factors that have role in the etiology of the disease:

- Physical characteristics of people, material and environment
- Socio-economic characteristics e.g., age, education , marital status, number of children and income
- Behavior or practices of people, knowledge, and attitude and beliefs (KAP)
- Events that occur in population

We usually use for cross-sectional study a questionnaire to ask about a lot of things

Cross-sectional Design

* Explanation in the next slide



Previous slide explanation

in cross-sectional design, what we do is we study and collect data at a **certain point of time**, then we survey, questionnaire or interview a representative sample of this population, then we look for who has a certain disease (for example diabetes) in this population of interest, what is the proportion of who have diabetes and what's the proportion without diabetes , then we ask about risk factors for example: BMI as a risk factor .. What proportion do have high BMI among those with the disease, what proportion have low BMI among those without the disease, and comparing these proportion we can get into a conclusion whether the BMI Is a risk factor for developing diabetes..

And we do this with all types of diseases and then risk factors of diseases.

Cross-Sectional studies examine a point in time

Cross-sectional Studies

Also called prevalence studies

- Are the simplest form of observational studies.
- Often used to study conditions that are relatively frequent with long duration of expression (nonfatal, chronic conditions) Can't work for diseases with short duration
- It measures prevalence, not incidence of disease
- Example: community surveys
- Not suitable for studying rare or highly fatal diseases or a disease with short duration of expression (Explanation in the next slide)

Not suitable for studying rare disease, why? **Because if we take a sample of population at one point of time, we won't find many people with this rare disease**

Not suitable for studying highly fatal diseases, why? **Because anybody who has been affected previously should've died and now won't be found in the sample that we've collected at this point in time**

Not suitable for studying diseases with short duration of expression, why? **If we survey sample of the population for example common cold or influenza that has short duration - a week or less - at the point in time when we collect data, we won't find so many people with influenza or common cold, because people who had influenza last week, now they're healthy, and many of those had it in the weeks and months before and don't have it now. So we can't get them in number in the sample that we collect now, we only can calculate who are currently having the disease**

sCross-sectional studies involve point prevalence, not incidence. For very infrequent diseases they are of limited utility

Cross-sectional…

Advantages of cross-sectional studies

- Less time consuming
- Less expensive
- Provides more information (lots of variables)
- 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.

Cross-sectional studies

Disadvantages

- **Weakest observational design, (it measures prevalence, not incidence of disease). Prevalent cases are only the survivors.** We have no idea about people who got the disease and got cured or died in the previous days, weeks or months.
- **The temporal sequence of exposure and effect may be difficult or impossible to determine.** (temporal sequence: the exposure should come before the disease (the effect))
- **Usually don't know when disease occurred**
- **Rare events a problem** (we can't study rare diseases).
- **Quickly emerging diseases are also a problem** (short duration diseases can't be used in cross-sectional studies).
- **Least useful in establishing causation.**

Is Cross-sectional design Descriptive or Analytical?

Dr. Sireen considered cross-sectional design as analytical in this lecture

- It may be difficult to decide whether the disease or the exposure came first, so causation should always be confirmed by stronger studies.
- The collection of information about risk factors is retrospective, running the risk of recall bias. **(this is a problem of cross-sectional design)**
- In practice cross-sectional studies include elements of both descriptive and analytical design.

Any result that we get from cross-sectional study is a little bit weak, and causation should be confirmed later by a stronger design to go into a case control or cohort study, or experimental even!

Study Designs in Epidemiology

Case-control, Cohort and Experimental Designs

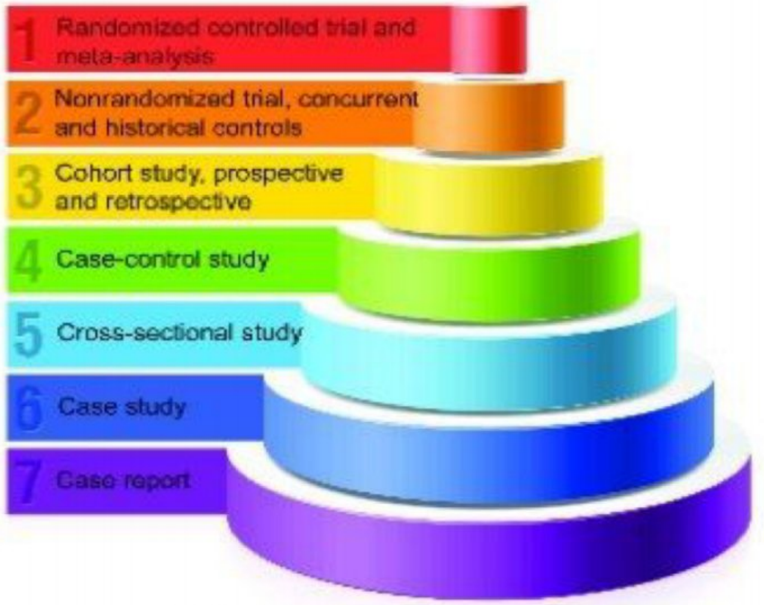


Figure. Hierarchy of Research Design

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Case-Control Study Design

Stronger evidence compared to cross-sectional

The investigator compares one group among whom a health problem is present (a group of people with the disease) 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.

We should have a group of women with breast cancer cases and another group without breast cancers who are free of the disease, we ask both about risk factor such as having obesity and we collect the information and look for compare the proportion of obesity and compare it between women with breast cancer to those without breast cancer.

e.g. A study to assess the effect of mothers' educational level on malnutrition among children

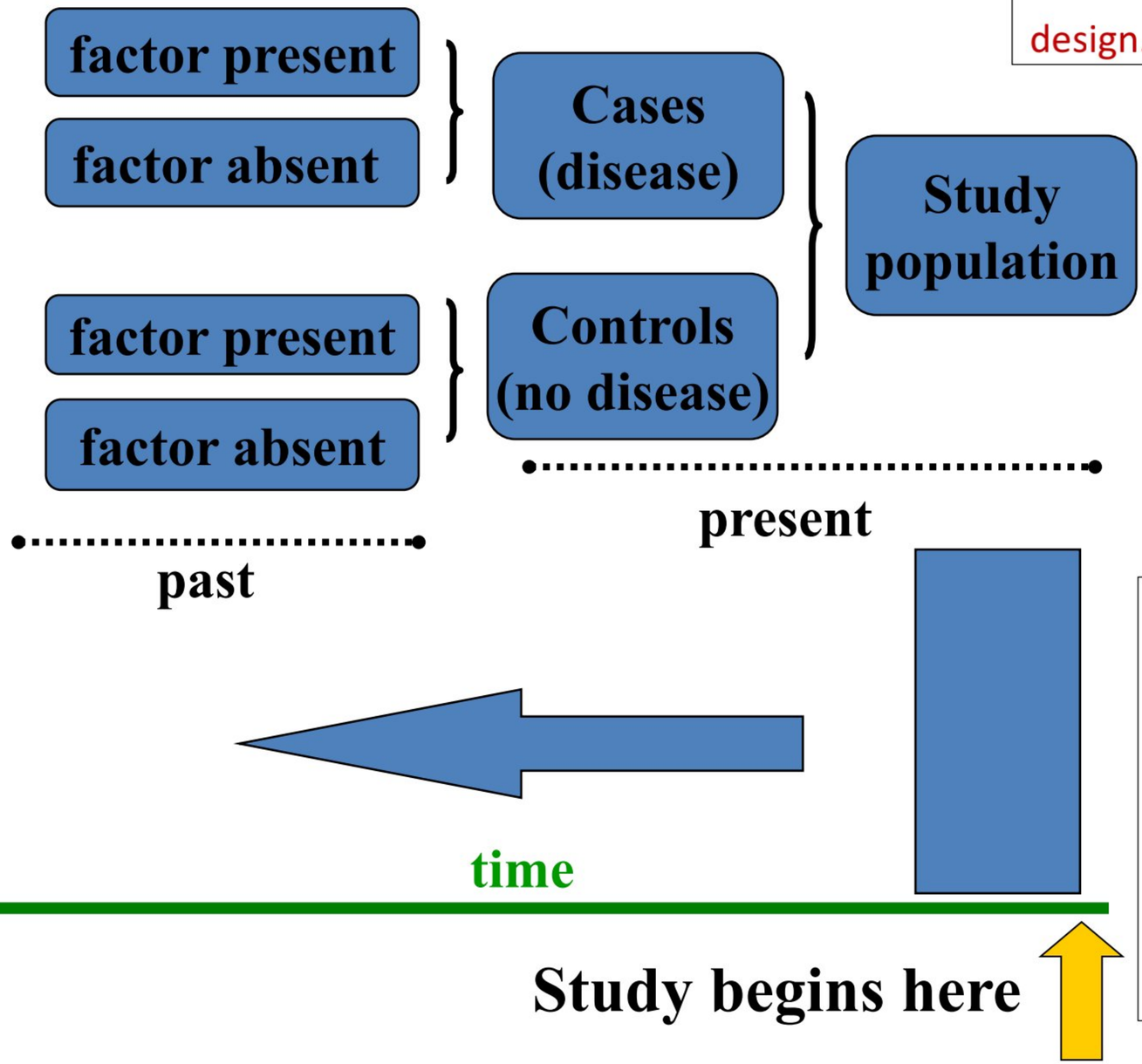
We get a group of children with malnutrition and another group without malnutrition (healthy) and we study the mothers' educational level, the proportion of mothers with high education among those of malnutrition compared to the proportion of mothers with high education among those without malnutrition. From comparing these proportions we can find out whether educational level of mothers is associated with malnutrition or not.

We start with study population from which we have a group of cases with a disease [EX: group of cases of lung cancer] and a group of control without a disease [EX: without lung cancer, healthy].

Then we look for the presence of a risk factors [EX: smoking], we ask them, see records or look for informations from all sources and identify what proportion of cases had exposure, which is smoking. At the same time we look for the control and see what proportion of the controls had smoking as a risk factor, and then by comparing the proportion of smokers among cases with a proportion of smokers among controls by doing statistical analysis we can conclude wheather there is an association or not.

Case-Control Design

This graph shows a sketch for case control design.



Starting point " in this timeline", we start here and we ask about historical or previous exposures and diseases.

Case-Control Studies

From doctors office and hospitals we can find a large no. of people who have been diagnosed even with a rare diseases. You can hold them and study them.

So, we have all records and phone numbers and contacts and when you come to the clinic you can see them , exam them and ask them about informations you need.

An “observational” design comparing exposures in disease cases vs. healthy controls from the same population.

- exposure data collected retrospectively. **From historical old data**
- most feasible design where disease is rare.

If we have a rare disease case control study will be an efficient way to study them. As we said before in cross sectional study is inefficient for a rare disease.

- This is the first approach to test causal hypothesis.

In cross sectional study is very weak in causal association. Much stronger in case control study.

- Definition of a case is crucial to a case control study.

It is very important to to make the diagnosis for the cases very specifically.

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).

We match each case with one or more controls which is similar in all aspects except for the absence of the disease.

- 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 to get our causal association..

Case-Control Study

Strengths:

1) Less expensive and less time consuming

Less expensive and less time consuming when compared to experimental and Cohort, but more expensive compared to cross sectional study.

2) Efficient for studying rare diseases

3) Allows the study of several different etiological factors for one disease.

EX: The cases that we have with lung cancer, we can ask about lots of risks factors, some of them we were to be related to disease and others maybe accidentlly discovered during this study. so we can study lots of risk factors.

We only collect all the data we need at one point in time and take all necessary information from patient, from medical records, blood test or any examination that we need to do at one point in time " do not use follow up".

4) No attrition problems (no follow-up)

It is a problem that we have other study need follow up and we start losing people during follow up period of the study if it was long. Some of people may emigrate, some of them die and others may lose intrest in participating in the study and get bored."this weaking the study"

5) Ethical problems are minimal (no risk to participants).

no intervetions no risk related to some exposure and treatments like other studies such as experimental.

Case control studies provide low cost answers to health questions

Case-Control Study

Limitations

1. Selection of an appropriate control group may be difficult.

Or very time consuming and has to be done very strictly.

2. Inefficient for evaluation of rare exposure

Efficient for rare disease not for rare exposure.
So those with a rare disease when we ask them about certain exposures, the exposure should be common among the cases not very rare.

3. Difficult to establish temporal sequence

This was a problem with cross sectional studies. Temporal sequence is very important for establishing causal association.
In case-control studies, we found people with the disease already and asked them about the exposures. So, we relied on their memory.
We can not be 100% sure about the fact that the risk factor came before the disease.

4. Determining exposure will often rely on memory, leading to bias (recall bias).

Recall bias: a difference in the way of remembering previous events between cases and controls.
People who have the disease tend intentionally to remember more deeply and specifically the different exposures that may be related to the disease (the risk factors), while when we ask the controls, who are healthy, they do not tend to remember a lot about the exposures

5. We cannot measure incidence & can only estimate the relative risk (RR).

That is a close estimate for the incidence.

Cohort Study

✓ which is stronger than a case control and stronger than a cross sectional

We start with the exposure

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 for new cases of the disease.

Both of them should be started healthy and free from diseases and one of them have exposure to risk factor and the other not. Then follow them up over time to monitor occurrence of new cases of disease. That's why we called it incidence study.

- 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?.

Because there is follow up into the future over time.

Examples explanation:

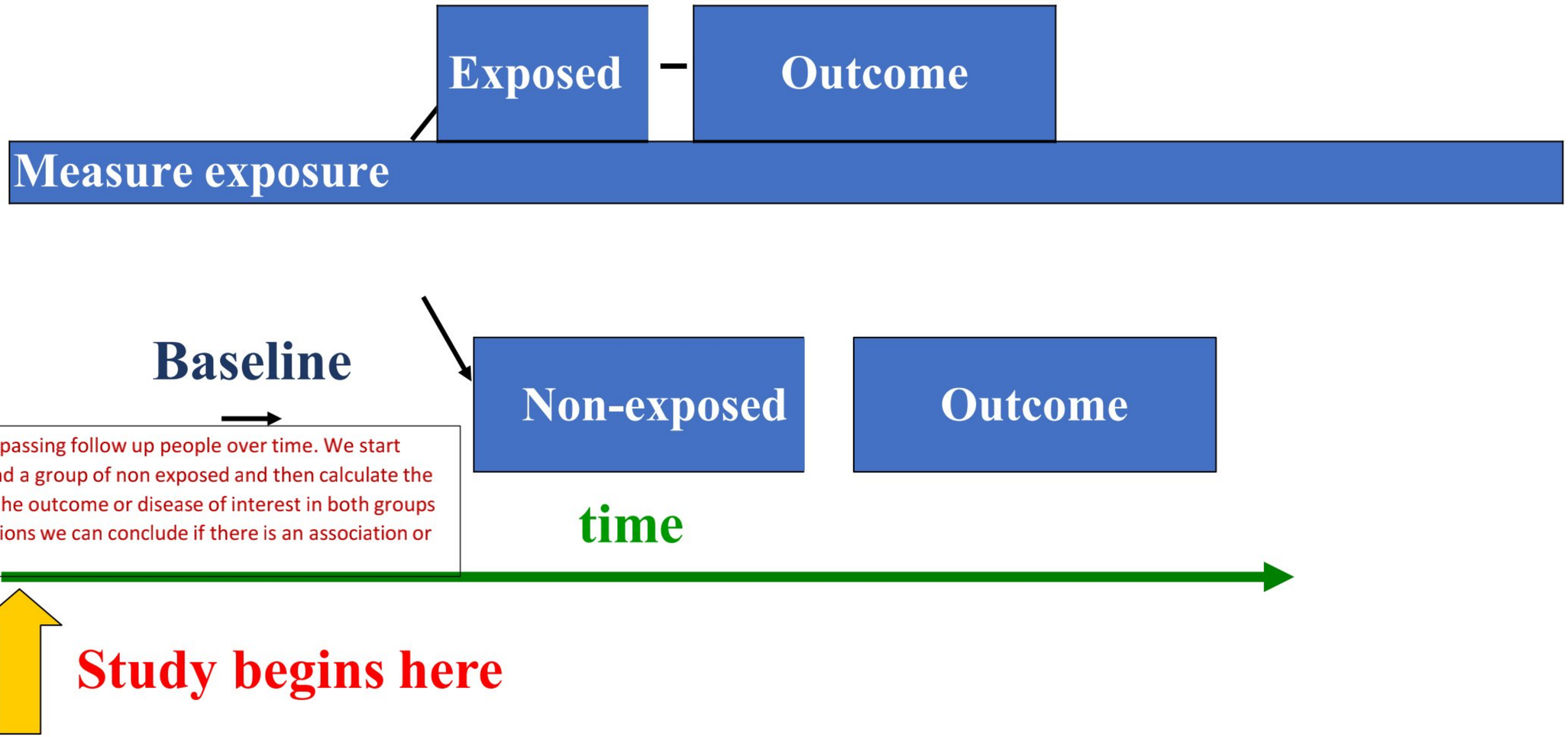
- **Does living in poor housing increase the risk of developing cancer?**
  we can have a group of people with the exposure living in poor housing and other groups similar to them but without living in poor housing, they live in better housing, and follow them up overtime and look for occurrence of new cases of cancer among the exposed compared to those without poor housing from that we can do statistical analysis and come up into conclusion whether living in poor housing is associated with developing cancer or not.

- **Does following a healthy lifestyle lower the risk of hypertension?**
  we can start with a group of people with a healthy lifestyle and another group of people with unhealthy lifestyle and follow them up overtime. We should start with healthy people with no hypertension, they should be diagnosed in a very specific way that they had no hypertension to see what proportion of those with healthy lifestyle will develop hypertension over the coming years (follow up is usually for years), and compare them with unhealthy lifestyle and see the proportion of those with unhealthy who developed hypertension and compare these proportions.

مثلا بهاد السؤال لازم اجيب مجموعتين من الناس ، وحدة منهم بتعيش بهاي البيوت ومجموعة تانية ما عاشت فيها واضل اراقب فيهم واشوف اذا رح تطلع حالات جديدة من السرطان بسبب مكان المعيشة او لا ، بعدين بقارن النتائج الي طلعت بعد المتابعة من الناس الي بعيشوا بالبيوت القديمة ومن الناس الي ما عاشوا فيها. وهيك بتطلع بمعرفة اذا المعيشة بهيك اماكن بتسبب السرطان او لا. طبعا لما نبدأ البحث لازم ما حد منهم يكون مريض بالسرطان.
والمثال الي بعده نفس الاشي بنجيب ناس بيتبعوا نظام صحي ونا ما بيتبعوا نظام صحي وبنتابعهم لفترات طويلة وشرط يكون ما معهم الضغط عشان نشوف الحالات الجديدة من الي بيتبعوا نظام صحي ومن الي ما بتبعوه وبقارنهم وبطلع نسبة بينهم

This graph shows a sketch for Prospective Cohort study

Prospective Cohort study



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..

Still, there are some situations where cohort study designs would be appropriate in the field. The classic design in a cohort study is shown here.

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 are tested between persons who had exposure to the possible risk factor at baseline and persons with no exposure.

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 with some retrospective at the beginning.**
  - ✓ Most of data is collected prospectively.

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

Cohort Study

Indications:

- When there is a good evidence of an association between exposure & disease.

that came from descriptive studies or from a previous cross-sectional study with some weak evidence if we are interested in studying more, we can do a cohort study.

- 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).

يعني لما بتقل نسبة الناس الي بينعمل عليهم البحث وبنسحبوا انسحاب المتطوعين لعدة اسباب مثل الهجرة او الوفاة او يمكن حسوا بالملل وما بدهم يكملوا البحث

Attrition: starting to lose people from our sample due to long follow-up period.
If they have a good environment for not losing them, for example, people working in a certain factory and exposed to a certain type of chemical, and we want to study the effect of this chemical on the long run over certain disease occurrence, we can know where to find them (in this type of industry or in this factory).

- When ample funds are available (it is expensive).

Because such study with follow-up and team who work with you, it will be expensive. You should have a good amount of funding available for the study.

Advantages of cohort studies

1. Valuable when exposure is rare
2. Examines multiple outcomes of a single exposures

You can look for multiple outcomes that may result from this single exposure. Maybe this outcomes have been anticipated before or discovered accidentlly and we don't know about it before.

3. Temporal relationship is known

We started with healthy people, some of them with the exposure and others without the exposure. Later over the coming months or years of follow-up, they developed the disease or health problem of interest.
Temporal sequence here is struggling established, we are 100% sure that the risk factor or exposure came before the disease, this is very clear and easy to establish.

4. Allow direct measurement of risk

Or incidence "measure strongly"

5. Minimize bias in ascertainment of exposure **that's make the result much stronger**
   - ✓ 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

   It will not be efficient in terms of money and time to wait for 20 or 30 years for occurrence of a long-lasting chronic disease like cardiovascular disease for example, or for a disease that is veeeery rare in population “the incidence of these disease is very low”, we will have to follow so many people over very long time to get some cases.

4. Loss to follow-up is a problem

   we’ll start to lose so many people from the sample over the long follow-up period.

Framingham Study

✓ Framingham heart study is the most famous Cohort study in the world.

What is the Framingham study?

When did it start? Where?

What was the disease studied?

What are the most important findings?

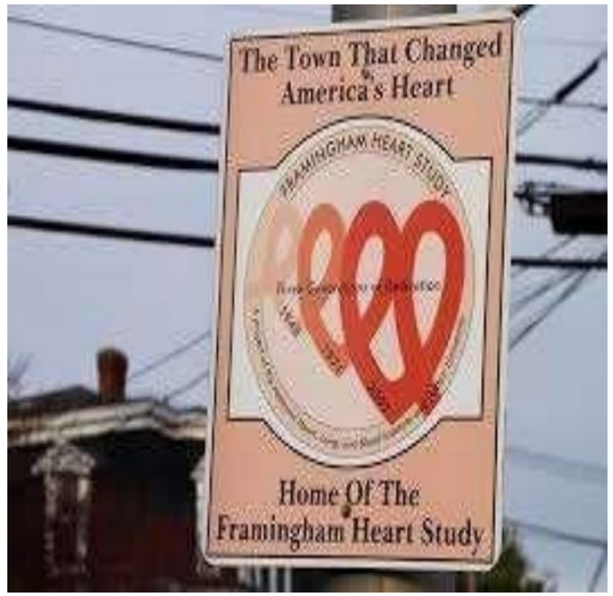
How many people participated?

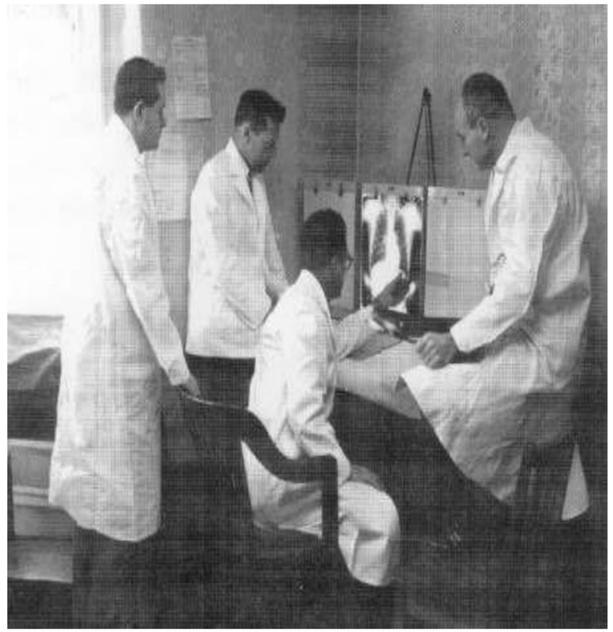
How many generations?

When did it end?

So, for how long did it go?

حسب ما فهمت من حكي الدكتورة انه هدول الاسئلة يعتبروا اسايمنت لالنا وداخلين بالفاينل





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

The intervention or the exposure usually is a new therapeutic treatment or medication or a new surgical procedure.

EX: new surgical procedure that hane been recently invented and need to try it then see the effect on a certain outcome [ex. Length of stay in hospital after a certain surgery, or a therapeutic treatment for getting relief from the disease}

Types of experimental studies:

Strongest type

1. **Randomized Controlled Trial**: on patients in clinical settings **(e.g. RCT).**

like fumes and smoke from the volcano eruption

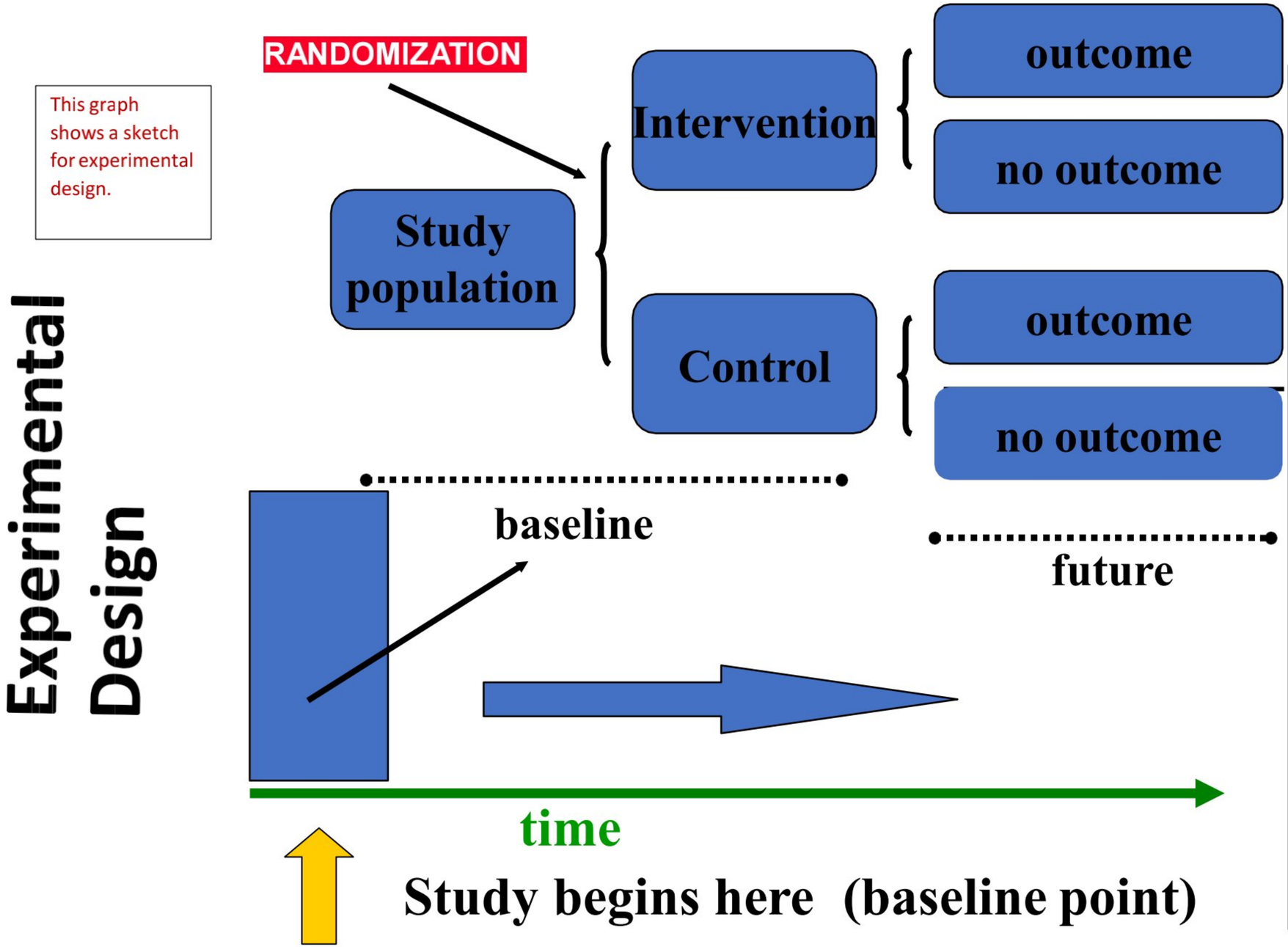
2. **Quasi-experimental:** Natural experiments, Field trial, Community trial (new Covid-19 vaccine), cross-over studies.

For example, now we are looking forward to receiving that new vaccine for COVID-19 and millions of people all over the world will start receiving the vaccine this will be like a field trial or community trial.

Experimental studies are the ultimate form of design in assessing causality as there is random assignment to groups.

This graph shows a sketch for experimental design.

Experimental Design



we start the study at this point in time where the arrow yellow is, there would be follow up into the future to see what happens. We start at the baseline with the study population, from which we take an intervention group that will take a new intervention and the control group (comparison group) that will not take the new medication or procedure. People will be allocated into an intervention group or control group for randomly, this is where we do **randomization.** So, the investigator will not choose in his own way who goes into intervention group (it should be randomized). Then we follow up intervention and control groups into the future and look for the outcomes, we see the proportion of people in the intervention group who will get the outcome and compare it to the proportion of people in the control group who get the same outcome, this is all in the future and from there we can do the statistical analysis and look for an association.

Experimental and observational studies

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.

From Wikipedia, the free encyclopedia

http://en.wikipedia.org/wiki/Statistics

RCT (Randomized Controlled Trial)

Randomized Controlled Clinical trials are the most well known experimental design. Provide the strongest evidence.

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

Controlled means: The researcher manipulates situations/objects.

→ have control over everything.

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** (ultimate means: provides most convincing evidence that should overrule any evidence from any other type of design that is weaker than the RCT).

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 of the participant, and use of Placebo.'those give the strength for the study'

بهاي الدراسة بهمنا العلاج الجديد مثلا وبنصير نجرب فيه ، بهاد المثال بحكيلي عن مرض معين ، بختار مجموعيتن عشوائيا مجموعة بعطيها العلاج الجديد ، والمجموعة الثانية بخليها عالعلاج القديم وبتابعهم لحتى اشوف النتائج وبعديها بقارن المجموعتين ببعض الي اخدت الدوا الجديد والي ضلت عالقديم.

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.

Explanations:

e.g. The effectiveness of a new treatment for rheumatoid arthritis.

We will take groups randomly, assign a group who will take their new medication and the other group (control group) should take the old medication, and then watch for a certain outcome, and see the difference between those with a new medication and compare them with the control group.

e.g. Comparing the length of stay in hospital between laparoscopy and surgery for appendicitis.

In the older days they used to do open surgery for appendicitis, then laparoscopy (الجراحة بالمنظار) was introduced. If you want to see if it's worth it to do laparoscopy (dose it make a difference in the outcome?), for example, if we say the most important thing is length of stay in the hospital after the operation, a group will have a new procedure that is laparoscopy, another group (control group) will receive the standard medication (or standard procedure) that is surgery for appendicitis. Follow people in both groups up and see the length of stay in days between those with laparoscopy compared to the stay in hospital with those with surgery and see to what extent does laparoscopy reduce length of stay, is there a significant difference between both procedures or not?

Randomization: random allocation of study subjects into 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 observation bias.

The more types of blinding used, the strongest is the results and more avoidance of observation bias.

Single: the patient does not know if he is in control group or treatment group (they are taken the new medication or not).

Double: both patient and the observing physician does not know if the patient is in control group or treatment group.

Triple: [strongest one] neither the patient nor the following physician or clinician nor the person who does the statistical analysis know if this person is in control group or treatment group.

Placebo is used as blinding procedure (where we use the placebo and the patient thinks that they are using the new medication) …..an inert material indistinguishable from active treatment. Used to avoid Placebo effect: tendency to report favourable response regardless of physiological efficacy that unintentionally happens with the participants in the study.

Randomized Controlled Trials

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 if exposure has well-known benefit.

That's like medication invented and developed for ADIS or HIV infected people when there was no treatment available and new medication that proved some efficacy, they tried to do randomized control trials on people with HIV infection in Africa mainly and that has very crucial ethical consideration. The issue was if the treatment was known to have high efficacy from earlier studies, so how could you deny some of the people with HIV infection the medication, those who will have the medication will most probably survive and live longer and get better and those who were denied the medication will most probably get worse and maybe die because they were assigned into a control group where an effective medication was available.

Understanding controlled trials: Why are randomized controlled trials important? By Bonnie Sibbald and Martin Roland

http://www.bmj.com/content/316/7126/201.full

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.

- ✓ If a randomized controlled trial comes up with new evidence, this should overrule any older results that have been produced by weaker types of studies, given that the trial has been carried out properly.

It is not unexpected to find that observational studies find different results than for clinical trials. For example there have been 100s of observational studies demonstrating that hormone replacement was protective for women. However, when this was put to a clinical trail, the surprising result was that hormone replacement was not protective

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

Semi- Experimental

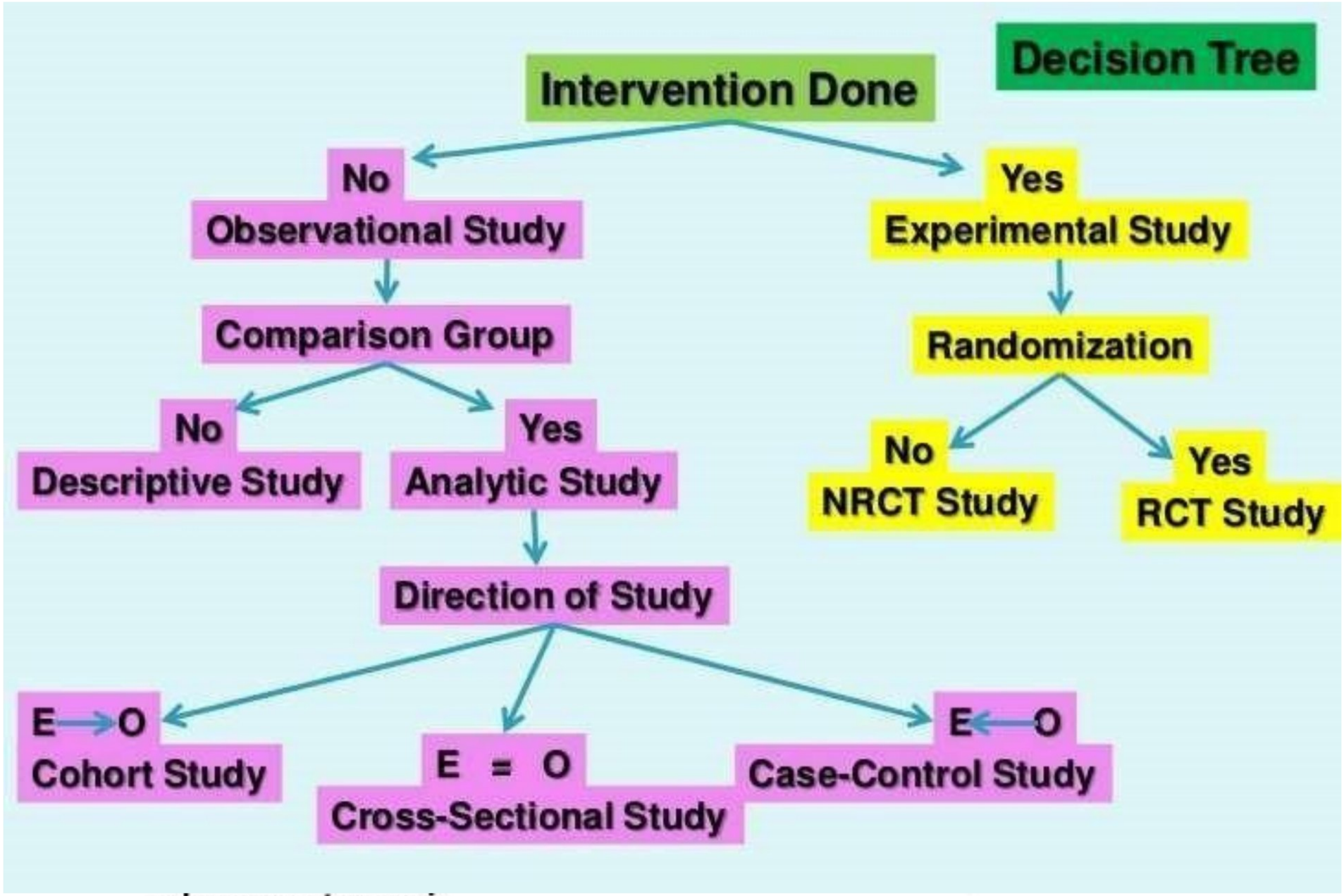
The researcher does not decide or plan the intervention (e.g. changes in using health care after removing ophthalmic services from health insurance), no Randomization or no control group”things that happened by themselves”.

Natural experiments

Factor occured naturally : e.g. Increase in mental disorders following an earthquack.

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

The person take byself the old medication and then the same person take the new one and compare the change in hypertension, for example, how it was controlled with the old medication and compare it to hypertension control with a new medication. We can also use this for a new type of pain relief medication, use the same person as a control for himself.



who are not anemic