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Summary
Week 2: Introduction to study design, surveys and questionnaire design

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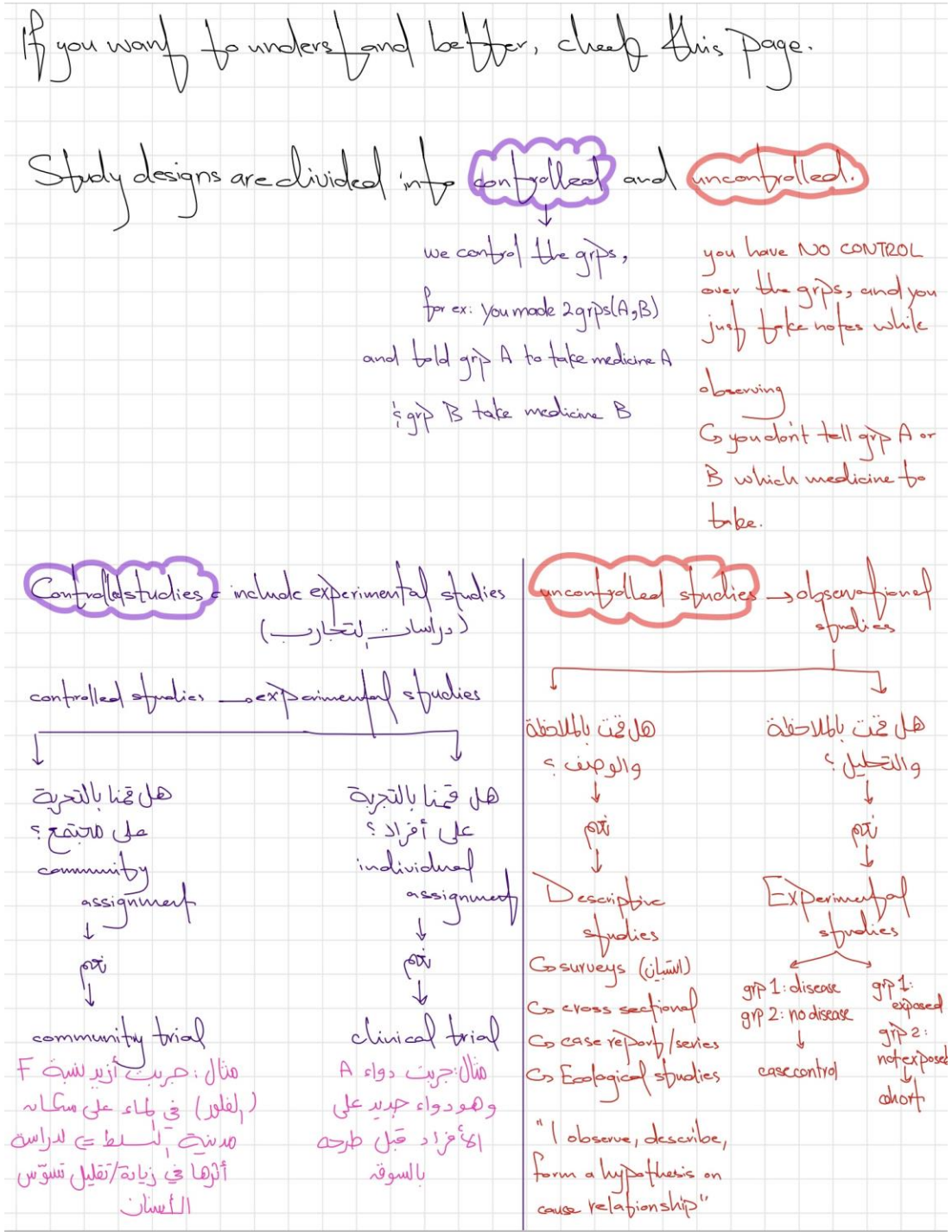
Objectives

- Understand the types of study design
- Compare prospective with retrospective studies
- Overview of surveys design
- Type of studies that can be conducted through survey design
- Questionnaires design and common problems in questionnaires wording
- How to deal with sensitive questions in questionnaires

- A nice beginning for the lecture , just to introduce you to the topics



Part 1: Introduction to study design

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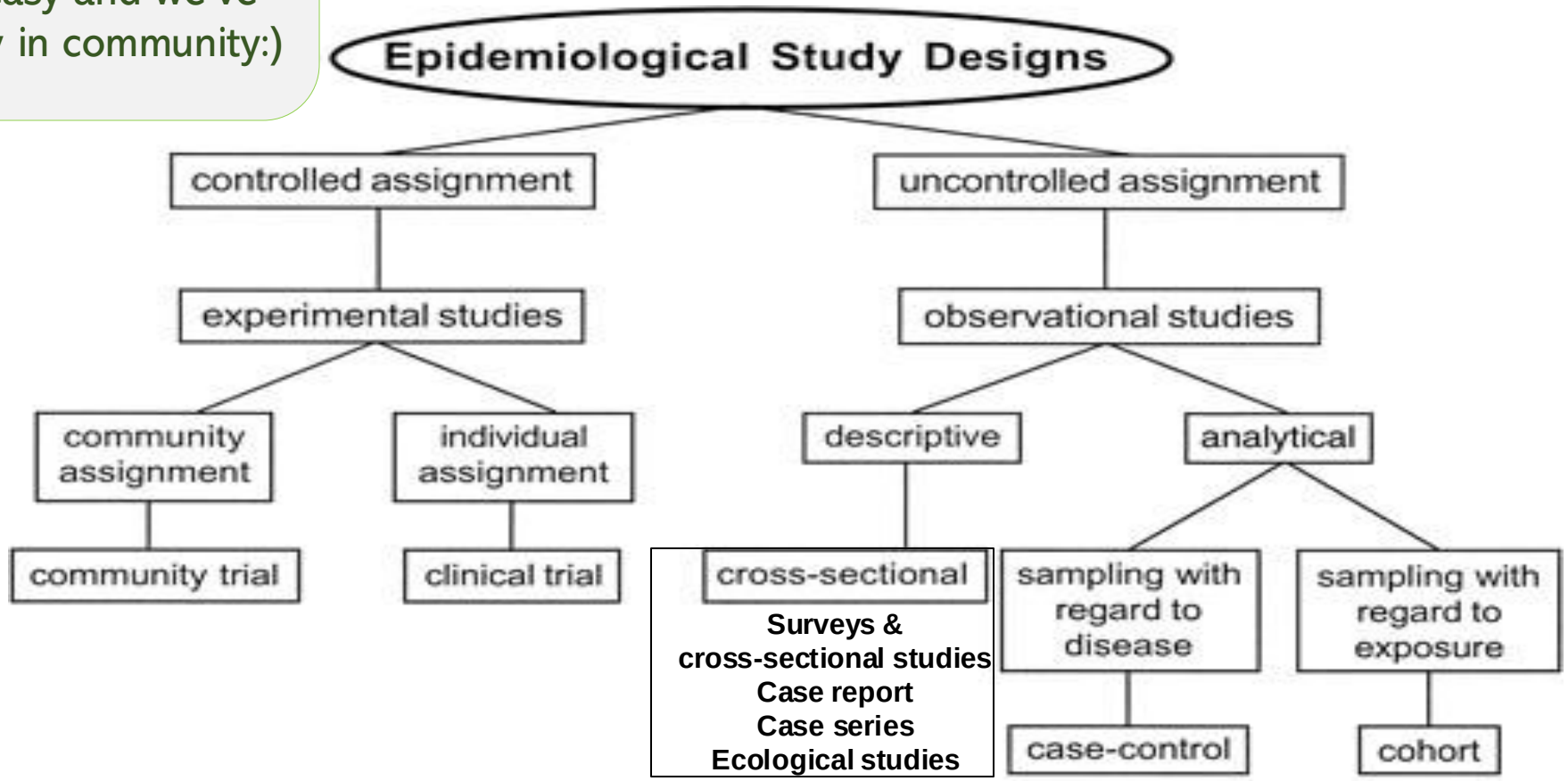
Study Design: Definition

A study Design is a specific plan or protocol for conducting the study, which allows the investigator to translate the conceptual hypothesis into an operational one.

- First of all, we have idea , research question and objectives , but how can we achieve the objectives in a valid scientific way? => Study design.
- Examples to use survey : you did a survey to collect patients attitudes or beliefs about a disease OR patients/family knowledge about a disease OR complaints of medications, , quality of life
- To look at the prevalence of disease => cross sectional study.
- Compare medication A vs B =>clinical trial

-Take a moment and read it quickly, we will explain every point in the next slides
-Don't panic, it's easy and we've taken it previously in community:)



Source: Waning B, Montagne M: *Pharmacoepidemiology: Principles and Practice*: http://www.accesspharmacy.com

Epidemiological Study Designs

controlled assignment

You have a rule as an investigator in term of what will the patient receives through out the investigation , how/where they are referred

Referral Process: Participants may be referred to specific locations for receiving treatment or undergoing evaluations. The investigator ensures referrals follow the study protocol, providing instructions on where participants should go for treatments, tests, or follow-up visits.

uncontrolled assignment

Any study which you as an investigator WILL NOT interfere with the participants in term of “types of medication , frequency of taking medication , referred system…etc. “
What you do is Observe and Report outcomes Prospectively OR Retrospectively.
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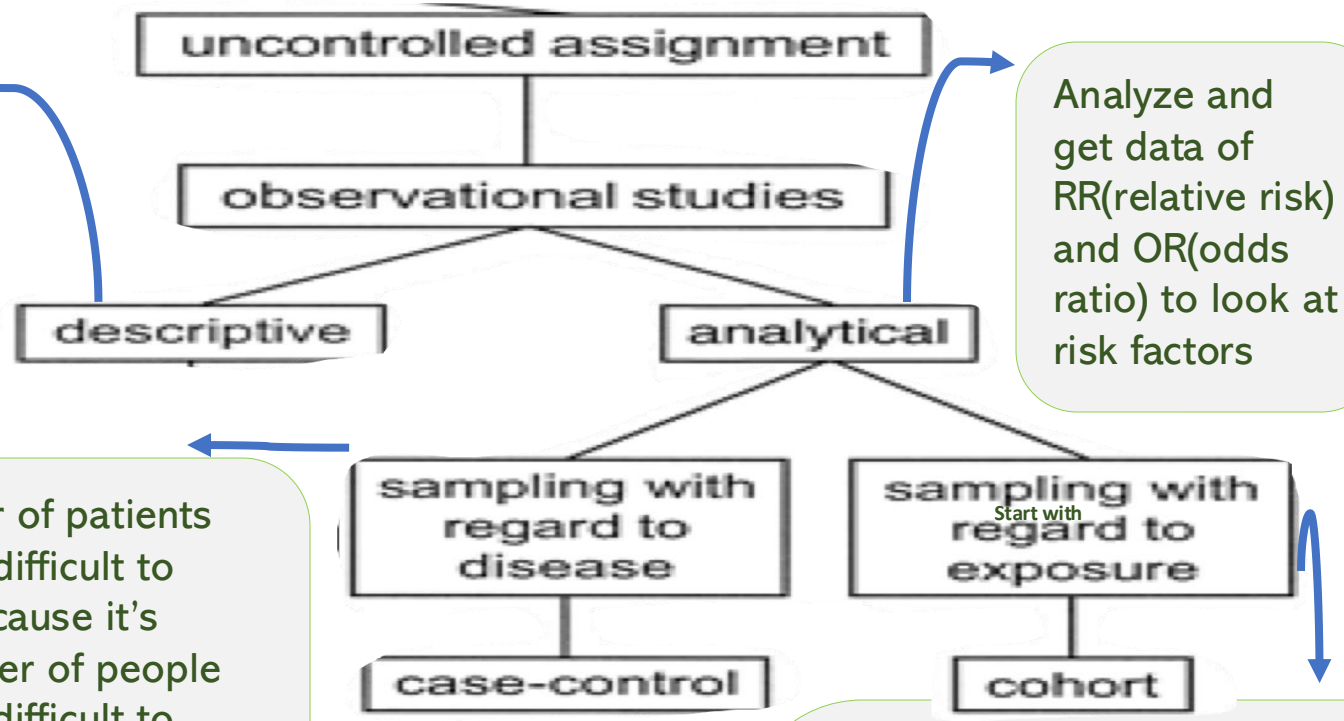
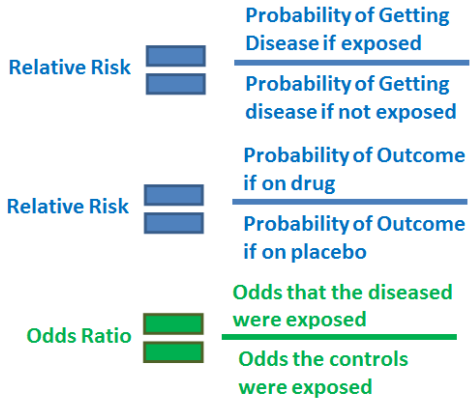
يعني ما نتدخل أبدًا في المعطيات ، احنا فقط بنلاحظ و ونسجّل



To discover the outcomes, so it is just for description and making hypothesis ,not for proving

Analyze and get data of RR(relative risk) and OR(odds ratio) to look at risk factors

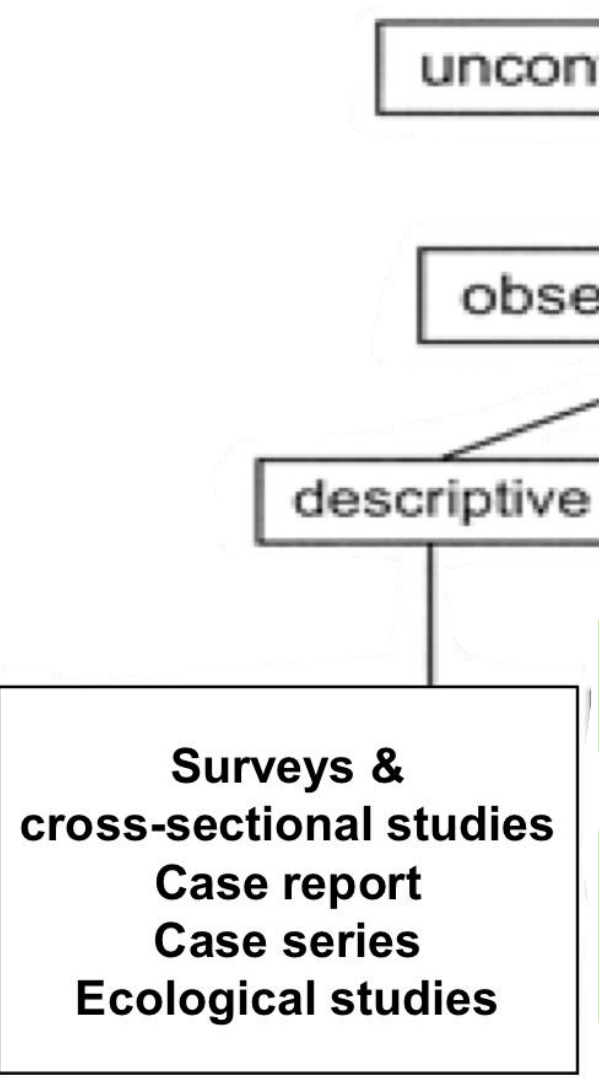
Extra picture



If you need a large number of patients with a rare disease =>it's difficult to conduct a cohort study because it's difficult to find large number of people with rare disease ,and it's difficult to follow them up .In this situation we do case – control study.

- For example, you want to study stomach cancer, and the incidence is for example 50 per 100,000 and you want to look at the risk factors. Here we use case control study. Case control is basically having two groups group 1 would be patients without the disease and group 2 would be patients with the disease. These-two groups should have people who are exactly alike in terms of age, gender health status, except for the presence of a disease .

The easy way to look for risk factor association
Let's take an example, we have group A,B .
Group A & B : no cancer , no ischemic heart disease, no hyperthyroidism
Group A : exposed to risk factor (smoking)
Group B : not exposed to risk factor (non smokers)
After 10-20 years later, we looked for cancer, IHD , hyperthyroidism among exposed vs non exposed
We found that 10% of group A had developed hyperthyroidism, while 2% of group B had developed it.
Now we can calculate RR (will be explained later) :
10% / 2% = 5 ,so smokers are at 5 times risk to develop hyperthyroidism



Surveys: questionnaires , asking about attitude, perception, complication and knowledge…etc.

Cross sectional studies: you will assess group of participants over a specific period to look at prevalence of different illness about the proportion of risk factors presence.

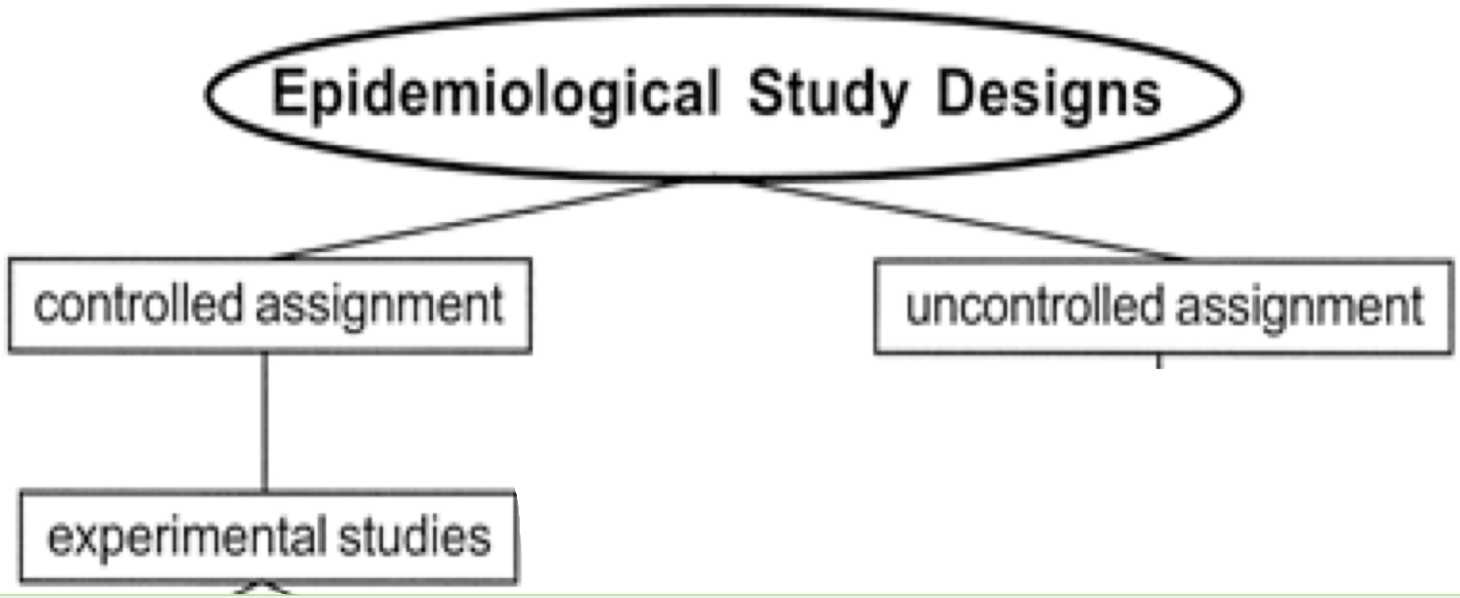
Case report: special form of surveys , they are done when you have a patient present with strange manifestations or adverse drug reaction to a medication or you have conducted a complicated operation that is rarely done and you wanna share the experience with the medical society

Case series: similar to case report, but on group of patients, these patients came with certain manifestations , adverse drug reactions, rare disease and you want to share their profile (for eg, symptoms or you investigations) to medical society you choose.

- Ecological studies: you draw correlation between different factors like group of countries with high red meat consumption and incidence of colorectal cancer, then you've found positive correlation. So you firstly generated a hypothesis " red meat could be a risk factor to colorectal cancer"

- Notice we've assumed that there is a relationship but we didn't confirm because this is a 'descriptive ' study

- All descriptive studies will help you find cause - effect relationship and it will help you in generating a hypothesis on risk factors
- Example : You have a group of patients with hypothyroidism and you knew that the prevalence rate of smokers is 30% among the hypothyroid patients while the general population or the patient in the study with normal thyroid function test has the prevalence 6%. This means that smoking could be a risk factor for hypothyroidism ,but what we need is to confirm whether this is a risk factor or not, so we need to do an analytical study



Example of experimental studies: You can compare drug A versus drug B or different dosages (dose A versus dose B). Another example is comparing the outcomes of patients with low back pain based on their referral timing to physiotherapy: So you can compare the outcomes of patients referred to physiotherapy at an early stage versus those referred at a later stage.

This study is considered experimental because the investigator chooses which patients will be referred early and which will be referred late , and this is a clinical trial where you assess outcomes such as pain levels and quality of life in patients, comparing the results of early versus standard or late referral.

- The controlled study is not just about comparing treatment A to treatment B but also explores different management strategies, such as the timing of the intervention.

More details in the next slide

Examples of clinical trial – Experimental studies :

Let's say you are conducting a study comparing different treatment approaches:

Surgical Intervention: You could compare outcomes between patients undergoing laparoscopic cholecystectomy and those having open cholecystectomy.

Diabetes Management: Alternatively, you might study patients with type 2 diabetes managed at an internal or family medicine clinic versus those referred to a tertiary or specialized diabetes center.

In controlled studies, it's not just about experimenting with medications. The key aspect is the role of the investigator, who can adjust various factors such as dosage, the site of referral, and management strategies…etc.

Uncontrolled Assignment Example

If you want to study the impact of aspirin intake on the incidence of colorectal cancer, you might use an uncontrolled design as follows:

You review hospital pharmacy records to find patients already taking aspirin, whether for the prevention of ischemic heart disease or other reasons.

These patients are then followed up to observe the incidence of colorectal cancer. In this case, you do not control the dosage or prescribe aspirin; you simply observe what happens.

Since the patients are already on aspirin, you are only recording outcomes, making it a cohort study designed to determine the incidence (uncontrolled).

Controlled Assignment Example

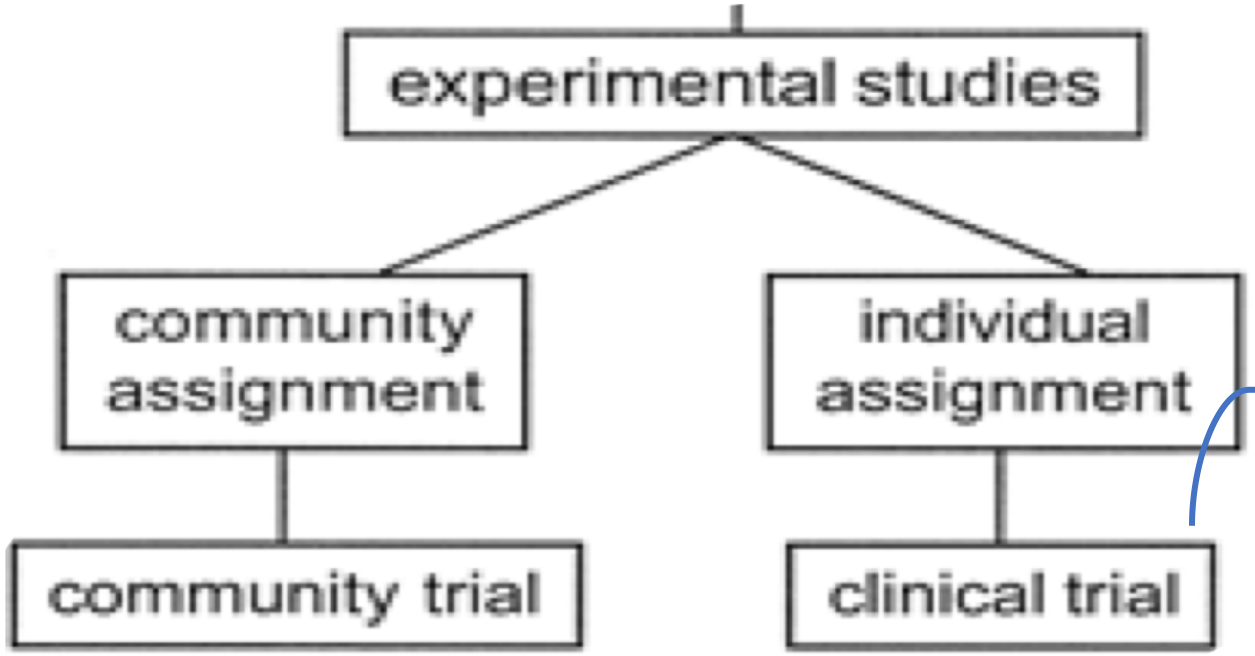
If you have a group of patients and you randomize them into different groups:

One group takes aspirin while the other takes a placebo. You then monitor the incidence of colorectal cancer over a period of five or ten years.

This would be a controlled study because you are actively assigning aspirin to participants who were not previously taking it.

For further control:

You could adjust the dosage or frequency of administration. For instance, you could have one group receive 325 mg of aspirin daily and another group receive 100 mg twice daily. Here, you are actively controlling the dosage or administration frequency to study its effect.



Like referring a group of patients with diabetes to either special clinics OR primary health care

Community Trials

In community trials, the focus is not on individual participants but on entire communities. For example:

Smoking Cessation Programs:

Suppose there are smoking cessation programs implemented in two different cities, Irbid and Mafraq. This would be considered a community-based assignment because the programs target the community as a whole rather than individual participants.

Reducing Dental Cavities Through Water Fluoridation:

A classic example of a community clinical trial is aiming to reduce the incidence of dental cavities in two cities, such as Salt and Aqaba.

The intervention involves increasing the fluoride concentration in all water supplies going to Salt, including tap water and bottled water, but keeping the levels within accepted safety limits.

In contrast, the water supply in Aqaba is left unchanged.

After five years, the incidence of dental cavities, especially among children under the age of five, would be compared between Salt and Aqaba.

In this type of study, there isn't a specific group of patients being treated or observed; instead, the entire community is considered as the target population.

Let’s summarize the main ideas ☺

Study Design	Description
Cohort Study	Follows a group of people over time to see how exposure affects outcomes, comparing those exposed to a factor vs. those not exposed.
Case-Control Study	Compares individuals with a disease (cases) to those without it (controls) to identify past exposures.
Cross-Sectional Study	Observes a population at a single point in time to assess the prevalence of outcomes or exposures.
Case Report	A detailed description of a single patient’s condition or treatment.
Case Series	A collection of case reports involving multiple patients with similar conditions.
Ecological Study	Examines population-level data to explore the relationship between exposure and disease outcomes.
Clinical Trial	An experimental study where participants are randomly assigned to receive a treatment or a placebo to evaluate its effectiveness.
Community Trial	A study where entire communities are assigned to receive an intervention to assess its impact on population health

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أتيأس الآن إذ قاربت الخطى الوصل ؟
هانت هانت ، الباقي خفيف :)

Controlled versus uncontrolled assignment

- Aspirin for prevention of colorectal cancer
- It can be a cohort study: Uncontrolled assignment for patients who are taking Aspirin for different indications
- It can be a randomized controlled clinical trial where we allocated patients to take Aspirin or Placebo

Observational epidemiology

- Provides information about disease patterns or drug use problems by various characteristics of person, place, and time.

- It also is used by epidemiologists to generate hypotheses regarding the causes of disease or drug use problems.

Observational epidemiology

a. Descriptive
Case reports and case series
Descriptive analysis (Person place time)
Ecological (correlational)
Surveys and Cross-sectional studies

b.Analytical
Case Control
Cohort **ONLY**

Note: sometimes, Cross sectional studies are considered ANALYTICAL, but the Dr prefers to consider it descriptive **ONLY,** BECAUSE only in certain cases cross sectional studies are used as analytical " it will be further explained later inshallah"
Note : When you look at the prevalence of a disease and the magnitude of its risk factors, it is descriptive, but if you then determined it as risk factors or causes ; analytical

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Descriptive
أمّا إذا حددت إنه هاي الأمور هي
Risk factors , causes
اذًا هي analytical

Epidemiological studies

- Observational studies are descriptive or analytical in nature.

- Descriptive studies attempt to uncover and portray the occurrence of the condition or problem, whereas analytical studies determine the causes of the condition or problem.
- Investigators in observational studies may plan and identify variables to be measured, but human intervention is not a part of the process.
- Experimental studies, in contrast, involve intervention in ongoing processes(=> By giving a treatment, referred (late / early)...etc.) to study any resulting change or difference.

Observational epidemiology

- Descriptive studies: provide insight, data, and information about the course or patterns of disease or drug use problems in a population or group.

- Analytical studies are used to test cause-effect relationships, and they usually rely on the generation of new data+ to see what are the risk factors and we can also look at the strong risk factors when we have higher relative risk " RR will be explained later inshallah"

Epidemiological studies

Clinical observation

Descriptive studies

He conducted case series then cross sectional study to compare women with /without Cervical cancer from the same hospital and from the community and found HPV positive was high in women with cervical cancer.

Analytical studies

Then he conducted retrospective cohort study and calculated Relative risk, then he confirmed HP.V. Is a risk factor for cervical cancer

Experimental studies

He then worked with pharmaceutical companies to improve a vaccine to prevent Cervical cancer.He managed to do that and a got a noble prize

Example : HPV + cervical cancer. Professor Hansen OBSERVED many patients with + HPV result had cervical cancer too !

HPV: Human Papillomavirus

Variation

Association

Association

What is the importance of observation?
Here is an example illustrating the importance of observations
- If a clinical trials were done on treatment A (new) on 1000 patients to look for outcomes and adverse reactions for that drug like hepatic failure .
Knowing that the incidence of hepatic failure was 1 / 100,00 for treatment A . Now in our study with 1000 patient it is hard to pick up these hepatic failure cases. Suppose that this drug was licensed, and people used it , if the patients who used that drug came to your clinic and you found liver impairment (hepatic failure) on these patients due to using that drug , you will help with your observation hundreds of people in and out your country

Does coffee causes pancreatic cancer

I am beginning to suspect that there is an association between coffee drinking and pancreatic cancer ……

I have seen a good number of cervical cancer patients positive for HPV… "professor Hansen question "

- Classical way starts with:

Case series

Descriptive analysis

Ecological study

Cross-sectional analysis

How to investigate this further?

Note : If you need to insert an intervention in the study => do a clinical trial

Prospective vs. retrospective studies

- Another important objective of this presentation is to differentiate between the prospective and retrospective studies , in prospective studies you'll start the follow up of the patient now ,you start going to the field to collect data about the patient. However, in retrospective studies you're gonna count on the existing records , as you review the patient's file and gather information from it, or you meet the patient like the case control studies and ask them about something in the past, about their exposure, about their intake and other potential risk factors

Prospective studies

You follow patients with certain risk factors, you collect information through cross sectional studies

- Watches for outcomes, such as the development of a disease, during the study period and relates this to other factors such as suspected risk or protection factor(s).
- The outcome of interest should be common; otherwise, the number of outcomes observed will be too small to be statistically meaningful (indistinguishable from those that may have arisen by chance).
- All efforts should be made to avoid sources of bias such as the loss of individuals to follow up during the study.
- Prospective studies usually have fewer potential sources of bias and confounding than retrospective studies.

You are going to do data prospectively , you have your data , forms, plan for lab analysis, diagnostic criteria for a certain illness.

Retrospective studies

- A looks backwards and examines exposures to suspected risk or protection factors in relation to an outcome that is established at the start of the study.
- Many valuable case-control studies, such as Lane and Claypon's 1926 investigation of risk factors for breast cancer, were retrospective investigations.
- Confounding factors (any factor that play role in risk factors and diseases) and bias are more common in retrospective studies than in prospective studies, sometimes we don't know what happened before the other, as for hypothyroidism and smoking , we don't know what happened before!

Confounding factors example 1:

A study was made to look at alcohol intake and incidence of lung cancer patients, the study found that the odds ratio was high and they concluded that where is high alcohol intake there is increase incidence of lung cancer, BUT when they analyzed the data , they regrouped the heavy drinkers into smokers vs nonsmokers . At the end they concluded that Smoking was the risk factor while heavy drinking was confounding factor

Confounding factors example 2:

If we did a study on hypothyroidism and made 2 groups
Group 1 in Amman = 500 females and 500 males , the prevalence =2%
Group 2 in Irbid= 800 females and 200 males , the prevalence =10%
We already know that females have higher prevalence to develop hypothyroidism, we can't say that Irbid has higher prevalence while the females in Irbid's sample were more!
Here gender is the confounding factor
Solution?
Get equal numbers of males and females in both groups OR equal proportions of females and males in each group

Comparison of Retrospective and Prospective Approaches

Retrospective	Prospective
- Inexpensive to conduct (you have the files , hire stuff to finish with you in a shorter time)	Expensive to conduct, to do interviews, follow up subjects
- Completed in a shorter time period	Completed over a longer time period
- Easier to access a larger number of subjects	More difficult to access subjects and usually requires a larger number of subjects
- Allows results to be obtained more quickly	Exposure status and diagnostic methods for disease may change, for ex, in cohort studies, for incidence of DM2 you might change the diagnostic criteria, machines…etc.
imp - Useful for studying exposures that no longer occur, ex: there was a medication that caused certain risk factor (caused congenital abnormalities in the fetus if the patient was pregnant) but it's now no longer available in the market, so the only way to study it is retrospectively by making the study on the children	Loss of subjects from the study over time may be substantial (**KEY LIMITING FACTOR**)
- Information and data may be less complete and inaccurate (**MAJOR LIMITING FACTOR**)	Information and data may be more complete and accurate (**STRENGTH POINT , less bias**)
-Subjects may not remember past information ,for example, in case control studies you are looking for risk factors for congenital heart disease and we ask the mothers about their medication intake in the first trimester or the diet intake or certain environmental factors and the ladies Told you that they can't remember so this is another limiting factor	Direct access to study subjects enhances reliability of data

Observational Epidemiology

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Additional sources

1. Book pages
2. Youtube videos
3. Webpages…etc

VERSIONS	SLIDE #	BEFORE CORRECTION	AFTER CORRECTION
V1→ V2			
V2→V3			

امسح الرمز و شاركنا بأفكارك لتحسين أدائنا !!