Observational Studies Lecture

Introduction

Clinical studies are done for a variety of reasons. They are commonly conducted to determine if a treatment is effective or more effective than another treatment, to determine prognosis of disease, to study how good a diagnostic test is, and to study potential risk factors and their associations with disease. We often want to know if something is helpful or harmful. Our own unsystematic observations in the clinic or in the hospital can be misleading because we only see a small, biased sample of people with a given problem. Studies can enroll a larger number of people and can be systematically conducted thus yielding "better" answers.

What are observational studies?

Studies of treatment, risk, and prognosis can generally be of two major types: observational and experimental. In **observational studies**, researchers just observe what happens to people. They don't intervene in any way other than measuring various things (like exposures and outcomes). In **experimental studies**, researchers perform an experiment and give one group in a study one "treatment" and the other group a comparison "treatment".

Why are observational studies performed?

This study design is performed for a variety of reasons. The most common reason is probably **convenience**. They can be quicker to conduct than experimental studies. Often databases from other studies or medical records or insurance records are just sitting around and can be easily used to answer new questions using observational methods. Sometimes it is **unethical** to study something using an experimental design. If something is known or strongly thought to be harmful a researcher cannot purposely give it to a study participant. It would not be ethical to purposely cause harm. An observational study is a great way to study potentially harmful exposures because the patient has been exposed of their own volition (and sometimes not) and researchers now just watch to see what effects the exposure has.

Observational studies can be of two types based on the time frame:

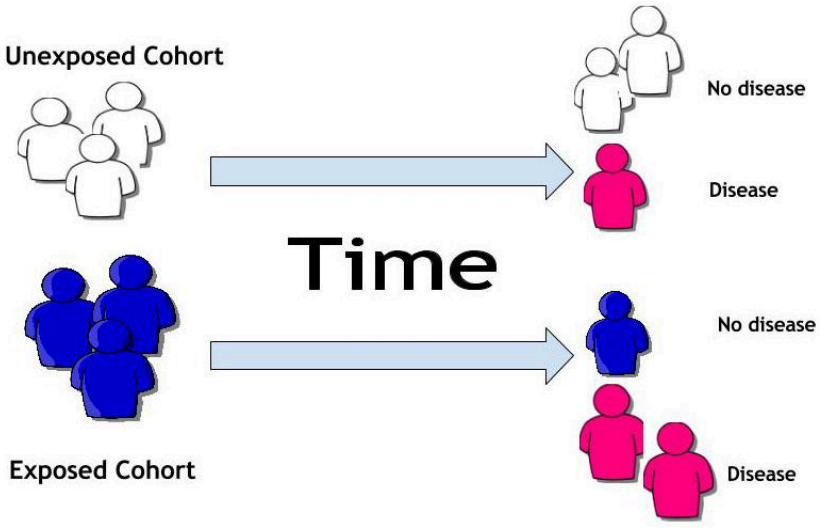
1. **Prospective**: study starts today and goes into the future
2. **Retrospective**: study starts in the past and goes forward either to today or into the future.

Observational studies can be of three types based on study design:

1. **Cohort**: follow exposed and unexposed cohorts for development of outcomes.
2. **Case-control**: study exposures that happened in the past to diseased and non-diseased patients.
3. **Cross-sectional**: often called a prevalence study. They can't determine time frames of when something happened. They can't determine cause-effect relationships. Commonly uses survey methods. We won't study these further.

Cohort Studies

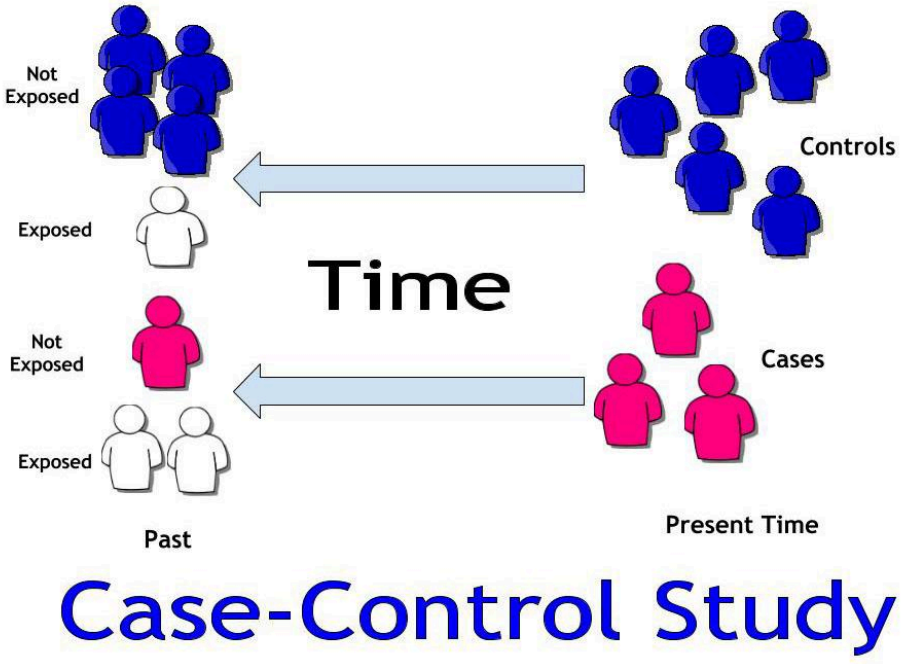
A **cohort** is just a group of people with a common trait. For example, the medical school is broken up into 4 cohorts: MS1 cohort, MS2 cohort, MS3 cohort, and MS4 cohort. Each cohort shares a common trait of year of enrollment in school. Cohorts are assembled in cohort studies based on exposure to something of interest. Thus, there is an exposed cohort and an unexposed cohort. **Exposure** is a general term for a person coming into contact with a risk factor which could be potentially harmful (e.g. tobacco smoke, high cholesterol, chemical spill) or potentially beneficial (e.g. statin, exercise). All participants in the study (whether in the exposed or the unexposed cohort) must be free of the outcome of interest at the start of the study. The exposed and unexposed cohorts are followed forward in time watching for the **development of outcomes**. Cohort studies can be retrospective (cohorts assembled at some point in the past) or prospective (cohorts are assembled in the present time).



Cohort Study

Case-Control Studies

Cases are people with disease and **controls** are people without disease. Case-control studies are backwards. They start by assembling a group of new cases and a group of controls and go backwards in time **looking for exposures** in both groups. The key thing (and the hardest part of the design) is to select controls that could have become cases (but didn't). They should be as similar as possible to cases but just not be a case. It is also challenging to assess exposure status in a case-control study because you are looking back in time (sometimes decades) to determine exposures. You have to rely on patient or family member memory, records (which were not developed for a study so they might be incomplete), or proxy measures (e.g. where a person worked at a plant as a proxy for possible exposure to a chemical). Case-control studies are always retrospective (sometimes you will see the term prospective case-control study. This is used when a case-control study is embedded in a prospective study). Case-control studies tend to be cheaper and quicker to run since all exposures and outcomes have occurred when you start the study.



Comparison of cohort and case-control studies

Cohort Study	Case-Control Study
Can establish **incidence** (or risk) directly	Can't determine risk directly
Follows clinical logic (exposure à outcome)	
Can look for **multiple outcomes**	Can look for **multiple exposures**
Good for **rare exposures**	Good for **rare diseases**
Not good for diseases with long latency periods	Good for diseases with **long latency** periods

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