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Scientific Medical Research

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In this lecture, we are going to talk about the second step in the research process ***Selecting a study approach*** 😊

- **Topics we are going to discuss: -**

A- Overview of study designs

- 1- Types of study approaches
- 2- Primary, secondary, and tertiary studies
- 3- Study duration
- 4- Primary focus: Exposure, Disease, or Population?

B- Case series

- 1- Overview
- 2- Case Definitions
- 3- Special considerations
- 4- Analysis

Overview of study designs
Types of study approaches

VIP ←

The design selected must be appropriate for the goals of the study. For example, if the goal is to see whether an intervention is effective, an experimental design is likely to be the only suitable one. If the goal is to understand populations, to describe patterns, or to ask research questions that are not focused on causality, the best design may be an observational one, such as a cross-sectional or cohort study. Often, the best study approach is the analysis of existing quantitative data rather than the collection of new data from individual participants. Sometimes the best approach is a systematic review or meta-analysis. Sometimes several different study approaches can be appropriate for exploring the relationship between an exposure and a disease.

In these situations, it is helpful to consider other factors during decision-making, including the expected duration and cost of the study, the populations available for inclusion in the study, and the possible availability of existing data.)

Other factors considered in selecting the study approach.

- Eight study designs are listed in the figure below {FIGURE 7-1}. Many research projects use variations of one of these approaches, and in others a hybrid of two approaches might be suitable.
- A diversity of designs can be valid and helpful approaches for the collection and analysis of new data, the analysis of existing data, and the reviewing the literature in the health sciences.

FIGURE 7-1 Summary of Study Approaches

Study Approach	Goal
Case series	Describe a group of individuals with a disease
Cross-sectional survey	Describe exposure and/or disease status in a population
Case-control study	Compare exposure histories in people with disease (cases) and people without diseases (controls)
Cohort study	Compare rates of new (incident) disease in people with different exposure histories or follow a population forward in time to look for incident diseases
Experimental study	Compare outcomes in participants assigned to an intervention or control group
Qualitative study	Seek to understand how individuals and communities perceive and make sense of the world and their experiences
Correlational (ecological) study	Compare average levels of exposure and disease in several populations
Review/meta-analysis	Synthesize existing knowledge

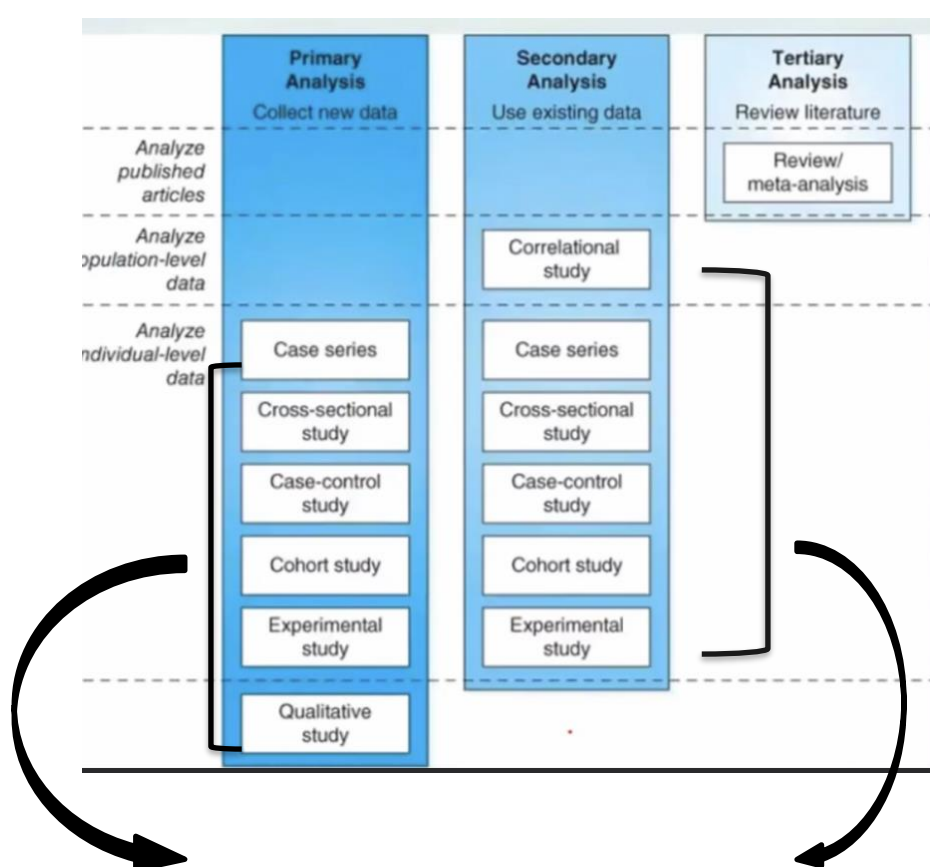
- **NOTES ON THE TABLE: -**

- Case series: the easiest study approach and could be one case or plenty of cases.
- Cohort study: It takes long time, so we need to see the patient multiple times to take observations.
- Experimental study: interventions could be medicines / educational programs etc)
- Qualitative study: For example: How do liver transplant patients feel about receiving an organ from someone else?? or heart transplant patients' feeling about waiting for a heart transplant?? (so, these can't be measured but they are perceptions).
- Correlational (ecological) studies: For example: heart failure is divided according to the NYHA (New York Heart Association) into 4 levels; so here we measure the treatment compliance in every level.
- Review / meta-analysis: for example: reading different articles about a certain subject and then you write down your own summary.

NOTE: DON'T WORRY ABOUT UNDERSTANDING THE DIFFERENT TYPES OF STUDY APPROACHES NOW, EVERY APPROACH WILL BE EXPLAINED 😊

Primary, secondary and tertiary studies

- A first clinical decision is whether to collect new data from individuals (a primary analysis), use existing data (a secondary data) , or write a review article (a tertiary analysis) {FIGURE 7-2} .



In both, the primary and secondary analysis, we can use any of the following approaches :-

1.case series / 2. cross-sectional study / 3. case-control study / 4. cohort study / 5. Experimental study

A qualitative study can only be used in primary analysis. Similarly, a correlational study can only be conducted in secondary analysis.

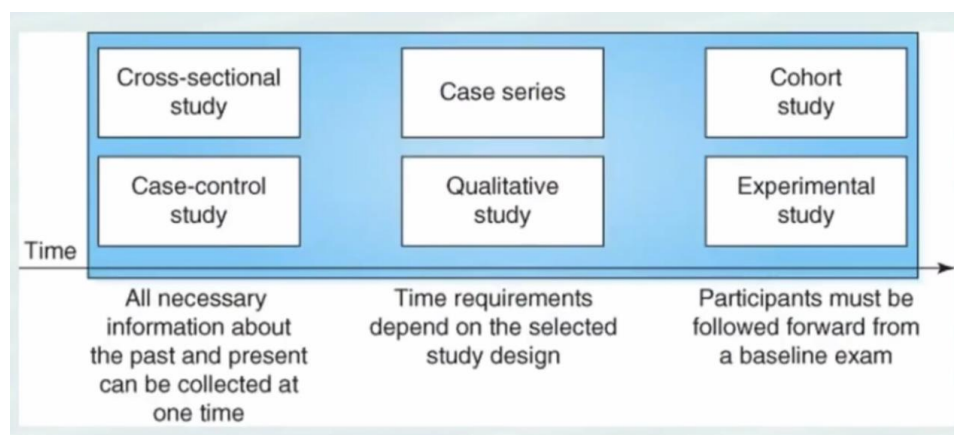
A- Primary studies are often time consuming because they require the collection of new data from participants. However, primary studies also give the researcher control over important details like the selection of a source population and the content and wording of the questionnaire. In addition to that, primary data has higher internal validity.

B- The obvious advantage of the secondary and tertiary analysis is that a researcher may be able to move quickly from the definition of the study question to the analysis of related Data. However, only a limited number of data sets and publications are available for analysis. Also, these sources might not include either the exact variables or the particular population of greatest interest to the researcher, thus affects you goal selection.

Study Duration

The time required for collecting and analyzing data varies from study to study.

- 1- Some **primary studies** call for the collection of all needed information from participants at one point in time. Others require participants to be followed for weeks, months or even years; according to the availability of the factors mentioned in page 1 {FIGURE 7-3}
- 2- The timeline for a **secondary study** might be very short if an entire data file and the relevant supporting documentation (such as copies of the questionnaire and codebook) can be downloaded from a website. Or a secondary data, collection might become labor intensive if old hospital charts have to be retrieved, read (often after deciphering somewhat illegible and faded handwriting), coded, and entered into a database.
- 3- The duration of **tertiary studies** is highly dependent on library access and on the number of publications that need to be acquired, read, and summarized.

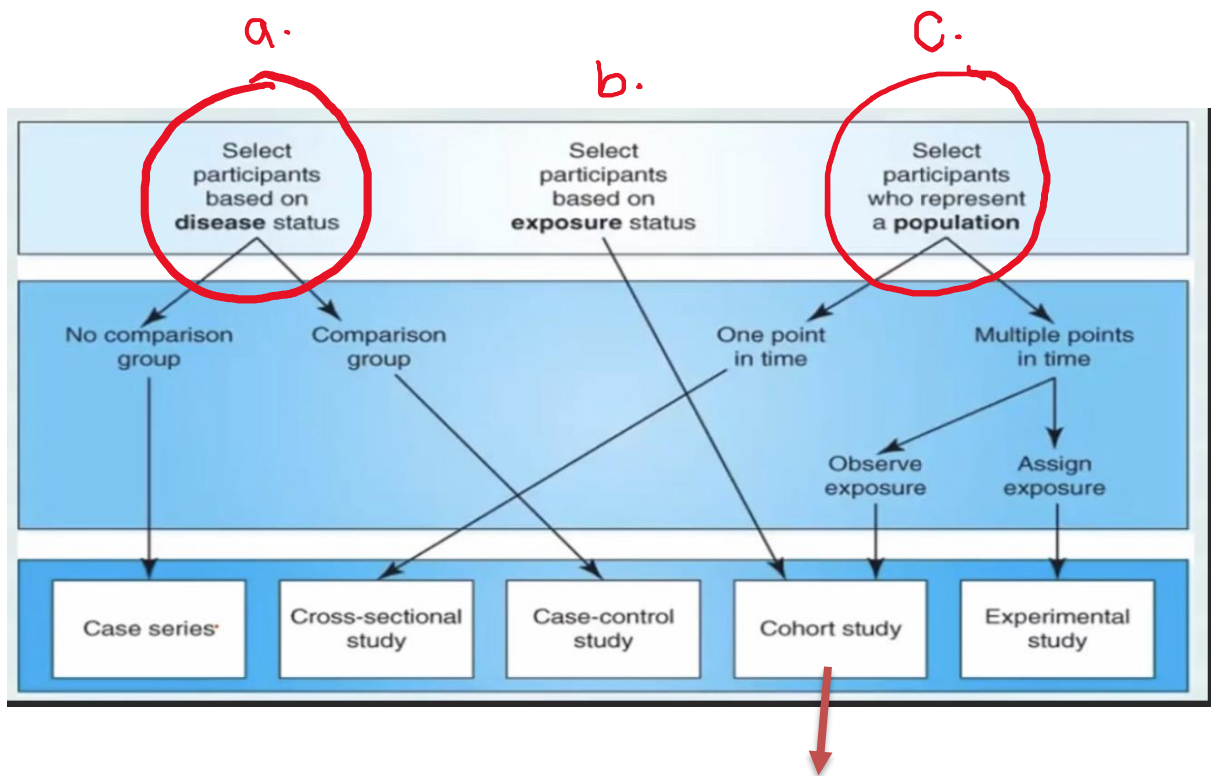


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Primary Focus: Exposure, Disease, or Population?

Most study designs are oriented toward a particular kind of population (Figure 7-4). All interventional studies and many cohort studies focus on individuals with a particular exposure—one that is assigned in experimental trials and merely observed in cohort studies. Case series and case-control studies both focus on individuals (“cases”) with a particular disease. Cross-sectional studies and some types of cohort studies seek to recruit a study population that is representative of a well-defined larger population. Researchers who have relatively easy access to a group of individuals with a particular exposure or disease or to a unique population group often choose a study approach based on the design’s appropriateness for the available participants.



Example on cohort study: -

Like a group of people working in a factory and they are exposed to hazardous chemicals \triangle and we want to study if there is a relation between the exposure to these chemicals and developing cancer.

- NOTE: pay attention to the difference between **a** (SELECTING PARTICIPANTS BASED ON DISEASE STATUS) and **c** (SELECTING PARTICIPANTS WHO REPRESENT A POPULATION) **they are different .**

Case series

Overview

- 1- **Case report:** Describes one patient (new or strange case)
- 2- **Case series:** Describes a group of individuals with the same disease or who have undergone the same procedure {FIGURE 8-1}

FIGURE 8-1 Key Characteristics of a Case Series	
Objective	Describe a group of individuals with a disease
Primary study question	What are the key characteristics of the cases in this study population?
Population	All individuals in the study must have the same disease or be undergoing the same procedure.
When to use this approach	A source of cases is available, and no comparison group is required or available.
Requirement	An appropriate source of cases is available.
First steps	<ol style="list-style-type: none"> 1. Specify what new and important information the analysis will provide. 2. Identify a source of cases. 3. Assign a case definition. 4. Select the characteristics of the study population that will be described.
What to watch out for	(A lack of generalizability)
Key statistical measure	Only descriptive statistics are required.

VIP: remember we talked about external validity.

-Because our studies are based on a limited number of individuals, results can't be generalized on the whole population, but it is a very good step to start.

- This is considered to be one of the limitations of this study approach "lack of generalizability"

- **A case series can only be written and disseminated when a researcher has access to an appropriate source of cases and when there is a compelling reason to write about those cases.**
- **This study approach can be useful for: -**
 - 1- Describing the characteristics of and similarities among a group of individuals with the same signs and /or symptoms of a disease
 - 2- Identifying new syndromes and refining case definitions.
 - 3- Clarifying typical disease progression
 - 4- Describing atypical presentations of disease or unusual complications from a treatment.
 - 5- Developing hypotheses for future research

VIP: -

- **Some case series for rare conditions may require only a handful of participants.**
- **Others may include several hundreds or even thousands of individuals.**

Case definitions

☺ A researcher conducting a case series must select one disease of interest, determine what will be new and interesting about the study, and identifying an appropriate and available source of cases.

☺ the next step is to establish a clear case definition that spells out inclusion and exclusion criteria.

1- Inclusion criteria: -

The individual met the characteristics required for the study

2- Exclusion criteria: -

The individual met the characteristics required for the study, but he has a condition inhibiting him from being included in the study.

Example: -

We want to pick a sample of heart failure patients from different levels. From the exclusion criteria (the individual is not above 85 years old {so if the patient is above 85 year, he will be EXCLUDED from the study}), also we have from the exclusion criteria (having no mental health problems).

- Participants may be selected from clinical locations that use **ICD codes**.
ICD codes: that is, diagnoses based on the international classification of disease, known more formally as the international statistical classification of diseases and related health problems). If so, **ICD** number can be part of the case definitions, but a code alone I rarely sufficient to cover all inclusion and exclusion criteria.
- A more comprehensive case definition will include a disease description plus any relevant **Person, Place, ant Time (“PPT”)** characteristics {FIGURE -2}.

NOTE: -

ICD codes don't include place and time; place and time affects the disease's variables that we are studying; due to the fact that analysis of the same disease differs from place to place and from time to time. So, in order to limit our variables, we define the time and place.

FIGURE 8-2 Sample Case Definitions

Category	Example 1	Example 2
Disease/ procedure Person	Whooping cough (ICD-10 code A37) (Any person with a confirmed case of whooping cough, defined as an acute cough of any duration with isolation of Bordatella pertussis from a clinical specimen) or (a cough lasting 2 or more weeks with paroxysms of coughing, inspiratory “whoop,” or posttussive vomiting and contact with a laboratory-confirmed case of pertussis)	Liver transplantation Adult patients (ages 18 and older at the time of transplant), excluding those who were not receiving their first liver transplant and those who received multi-organ transplants
Place	Residents of Big City whose diagnoses were reported to the Big City Health Department (which requires notification of all diagnoses of pertussis)	Patients who had transplant surgery at the Oakville Regional University Medical Center
Time	First sought clinical care between January 1 and March 31, 2016	Recipients of liver transplants between January 1, 2006, and December 31, 2014, who were followed for a minimum of 2 years post-transplant

Inclusion criteria for the disease

Special considerations

- A case series might be constructed from primary data acquired by interviewing cases about their experiences using a questionnaire and / or qualitative techniques. The data might be supplemented or confirmed with a review of the participants' medical records.
- Alternatively, a case series can be – and often is – based solely on secondary data, usually acquired from a review of patient chart.

☺ when medical records will be consulted as part of the data collection process, it is often helpful to create a questionnaire that guides the extraction of information from these files.

☺ one of the limitations of relying on patient charts is that they usually contain only information deemed at the time of examination to be clinically relevant. The medical information in the patient files is not recorded for research purposes, so records are unlikely to contain all of the information that researchers need. Many signs and symptoms, patient comments, and clinician observations are not routinely recorded, A data extraction tool should include space to indicate the absence of a desired piece of information in the record. During the analysis and interpretation stage of the research project, the researcher should carefully consider the amount and type of MISSING INFORMATION.

- Case series studies come with special requirements to protect patient privacy. All case series projects require approval by a research ethics committee , as well as informed consent (**informed consent**: means that this patient approved to be part of the study after explaining everything about the study to him , by signing or implied approval , and this approval should be without coercion and without temptation (giving money ...etc)) from participants and/or the careful use of existing records .
- Researchers must pay close attention to **protecting the identities of participants** (their names , their numbers or any other indicators, unless if the researcher wants to see the patient again, he should use a code number and write it on a separate paper or anything else but not on the questionnaire that collects the data). This is especially important when the disease or the procedure is relatively rare and/or when the place and time characteristics are so narrow that individuals familiar with the source community might be able to recognize the participants. In most situations, all potentially identifiable information must be removed prior to publication

- All clinicians and researchers whose work might be enhanced by the use of photography must adhere to patient privacy laws and regulations as well as the policies of the medical centers where they work. When photographs will be used as part of a published article or published presentation, the researcher usually must acquire informed consent from patients before images with potentially identifiable features are published. Documentation of consent may be required even when there are no identifying marks in the image that could reveal the participant's identity.

Analysis.

Most case study reports do not require any numbers beyond simple counts and percentages, but some may benefit from the use of well-defined measures of morbidity and mortality. For example, **the case fatality rate** is the proportion of persons with a particular disease who die as a result of that condition. (This is different from the **mortality rate**, which is the percentage of members of a population who die of any condition during a specified time period. It is also different from **the proportionate mortality rate**, which is the proportion of deceased members of a population whose death was attributable to a particular cause.) When the sample size is sufficiently large, statistical tests may be used to compare subpopulations of cases or to compare before-and-after measures for the same individual participants.

Although many case series studies do not have any time dimension, some follow patients for days, months, or even years. In this type of study approach, the case series becomes, functionally, a cohort study in which all participants are defined by their disease status. Chapter 12 discusses cohort study approaches.

GOOD LUCK.