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INTRODUCTION

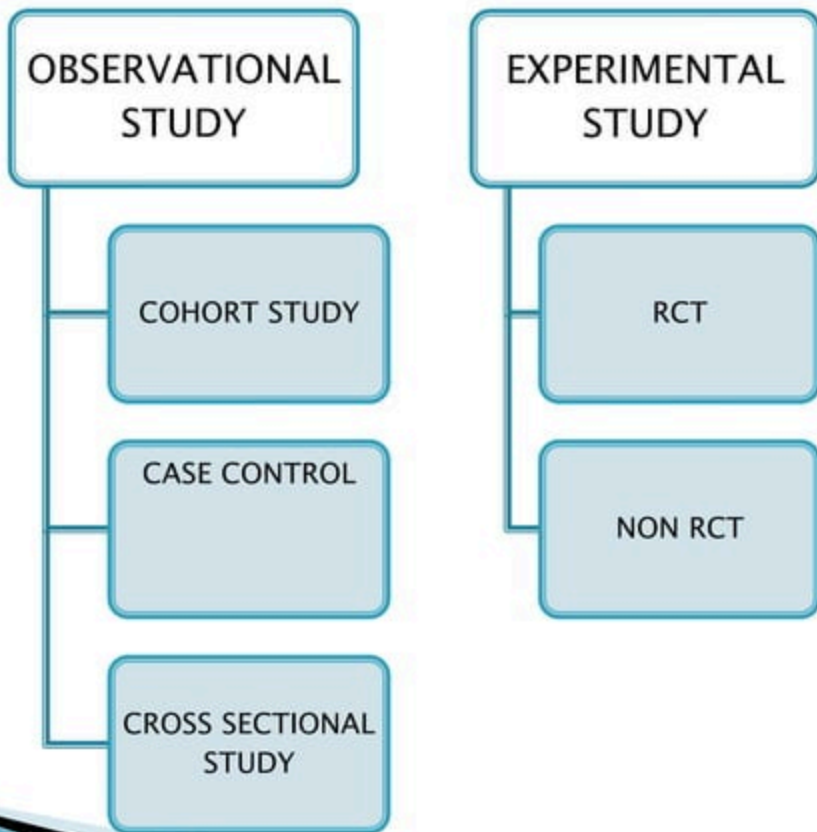
- ▶ **What is clinical trials?**

clinical trials (Human testing of a drug) are a set of procedure in medical research and drug development that are conducted to allow safety (or more specifically ,information about adverse drug reaction ,adverse effects and efficacy data to be collected for health interventions (e.g. drugs ,diagnostics,devices,therapy etc.)

• What are the different types of clinical trials?

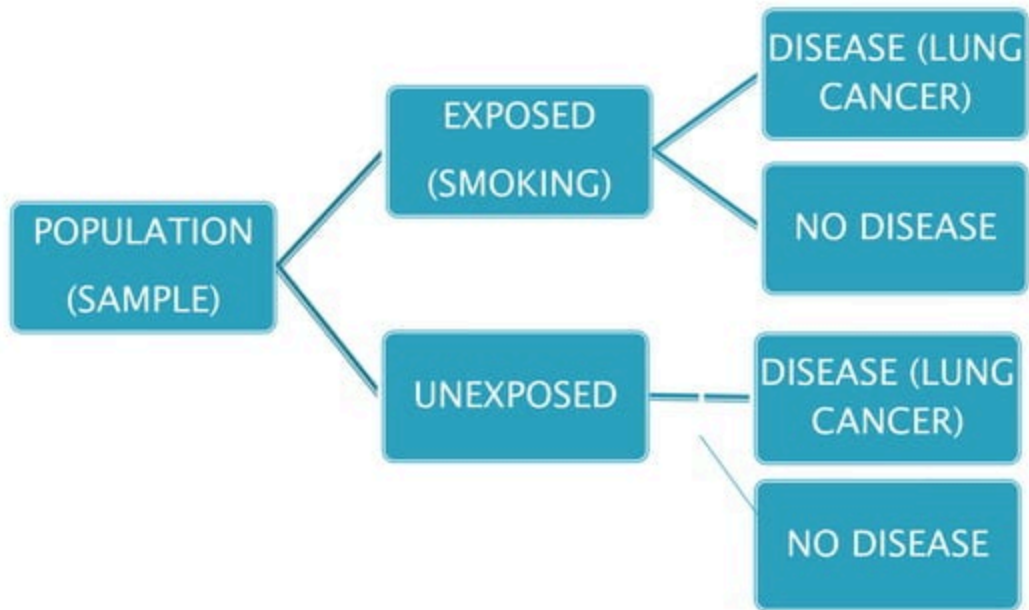
- ▶ **Treatment trials test experimental treatments, new combinations of drugs, or new approaches to surgery or radiation therapy.**
- ▶ **Prevention trials look for better ways to prevent disease in people who**
have never had the disease or to prevent a disease from returning. These approaches may include medicines, vaccines, vitamins, minerals, or lifestyle changes.

DESIGN OF CLINICAL TRIALS



COHORT STUDY

- ▶ A population at risk for the disease events is followed over time for the occurrence of disease and events.
- ▶ This study used to estimate how often disease or life events happen in a certain population.
- ▶ These are the best method for determining the incidence and natural history of a condition



STUDYS IS OF TWO TYPES

Prospective

- A Group of people is chosen who do not have the outcome of interest (for example, myocardial infarction).
- The investigator then measures a variety of Variables that might be relevant to the Development of the condition

Retrospective

- These use data already collected for other purposes. The methodology is the same but the study is performed.
- Outcome is already developed

Prospective

- ▶ Studies carried out from present time to future
- ▶ Can be tailored to collect specific exposure rate
- ▶ But long wait for events to occur
- ▶ Expensive
- ▶ Prone to high dropout rates

Retrospective

- Look at medical events from past to present
- Information is available immediately
- Difficulty in tracing subjects and doubt on quality of recorded information

CROSS SECTIONAL STUDY

- ▶ Is a type of observational study that are primarily used to determine prevalence.
- ▶ Prevalence equals the number of cases in a population at a given point in time.
- ▶ All the measurements on each person are made at one point in time.

	COHORT STUDY	CROSS SECTIONAL
Study group	Population at risk	Entire population
Common measures	Risks and rates	Prevalence

CALCULATING PREVALENCE

- ▶ The prevalence of a health outcome is simply the proportion of
- ▶ individuals with the health outcome in a population

$$\text{PREVELENCE} = \text{CASE} / \text{TOTAL POPULATION}$$

CASE CONTROL STUDY

- ▶ An observational study that compares patients who have a disease or outcome of interest (cases) with patients who do not have the disease
- ▶ Or outcome (controls), and looks back retrospectively to compare how
- ▶ frequently the exposure to a risk factor is present in each group

KEY POINTS

- ▶ Case control studies are usually retrospective.
- ▶ In this study the only outcome is presence or absence of the disease
- ▶ or whatever criteria was chosen to select the cases.
- ▶ Aim to identify predictors of an outcome
- ▶ Permit assessment of the influence of predictors on outcome via
- ▶ calculation of an odds ratio
- ▶ Can only look at one outcome

ADVANTAGE

- ▶ Efficient– saves time and energy
- ▶ Used for rare diseases, small sample sizes
- ▶ Can generate hypothesis for future study

DISADVANTAGE

- ▶ Susceptible to bias– recall, reporting
- ▶ Prone to methodological errors
- ▶ Selection of an appropriate comparison group may be difficult

EXPERIMENTAL STUDY

RCT

- ▶ An epidemiological experiment in which subjects in a population are randomly allocated into groups, usually called study and control.
- ▶ Groups, to receive or not receive an experimental preventive or therapeutic
- ▶ Patients are randomly assigned to the study all groups that help in avoiding bias in patient

ADVANTAGE

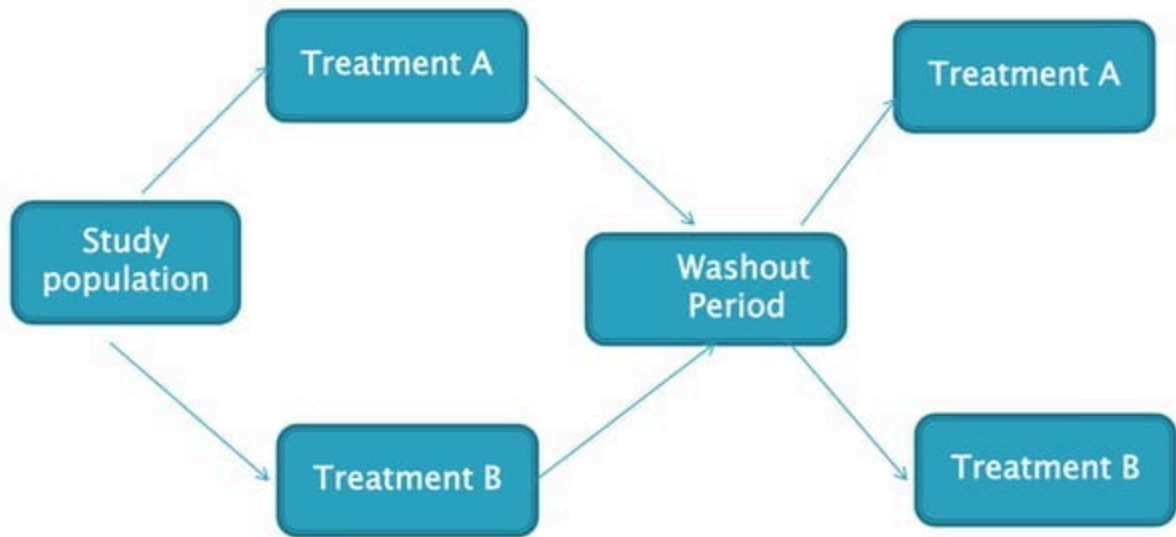
- ▶ **Comparative**
- ▶ One treatment is directly compared to another to establish superiority.
- ▶ **Minimizes bias**
- ▶ Randomization minimizes allocation bias and selection bias
- ▶ Blinding minimizes performance bias
- ▶ **Minimizes confounding factors**
- ▶ Randomization makes groups comparable according both known
- ▶ and unknown factors
- ▶ **Statistical reliability**
- ▶ Statistical test of significance is readily interpretable when the
- ▶ study is randomised

DISADVANTAGES

- ▶ Might demand vast samples size, which require more resources from the investigators
- ▶ Sometimes allocation of participants may be predictable and result in selection bias when the study groups are unmasked Trials are of longer duration and more expensive.
- ▶ Results may not always mimic real life treatment situation (e.g.
- ▶ Inclusion / exclusion criteria; highly controlled setting)
- ▶ Ethical limitations: some research cannot be ethically performed as an RCT (classically, RCT of the effects of parachutes on the survival of sky-divers)

Types of RCT

- Parallel group trial design
- Cross-over trial design
- Factorial design
- Group allocation
- Adaptive design



FACTORIAL DESIGN

- ▶ Studies involving two or more factors while randomizing are called factorial.
- ▶ Factorial design permits researchers to investigate the joint effect of two or more factors on a dependent variable.
- ▶ Used when it is desired to study the influence of a number of factors on the treatments compared as well as their interaction with different treatments factors on a dependent variables I designs

ADVANTAGE

- ▶ If no interaction, can perform two experiments **with less patients than**
- ▶ performing two separate experiments
- ▶ Can **examine interactions if this is of interest.**
- ▶ Cost: It is possible to evaluate multiple treatments within the same trial using
- ▶ fewer patients than individual comparisons

DISADVANTAGE

- Potential for adverse effects

Group Allocation Design

- ▶ Groups of individuals are considered as sampling unit and treatment is given to group rather than individual subject .
- ▶ Allows mass intervention
- ▶ It requires more patients than individuals design
- ▶ Necessitates cluster sampling method

Adaptive trial design

- ▶ An adaptive design allows modification /adaptation to trial and /or statistical procedure of ongoing clinical trials .
- ▶ Major adaptation of trial and /or statistical procedure of ongoing trials may results in a totally different trials that is unable to address the scientific /medical questions the trial intends to answer.

Non randomized clinical trials

- ▶ A clinical trials in which the particepents are not assigned by chance to diiferent treatment groups . Particepents may choose which group they want to be in,or they may be assigned to the groups by the researchers.
- ▶ NON RANDMIZED CONCURRENT CONTROL STUDIES
- ▶ NON RANDMIZED HISTORICAL CONTROL STUDIES

NON RANDOMISED CONCURRENT CONTROL STUDY

- ▶ Control group is predetermined (already selection of patients has done)
- ▶ Non randomized controlled trials rely on participant
 1. Volunteers to join the study
 2. Are geographically close to the study site

Thank you.....