Level 2 Semester 4

Module 7B

Research

Experimental studies

Instructor Name

- **Contact:.**

- **email:**

- **Mobile:**

- **Academic hours:**
  - ➢**……………day: 00:00-00:00 AM**
  - ➢**……………...day: 00:00-00:00 AM**

Reference:

Learning outcomes

At the end of the lecture, the students should be able to:

Why do you do research?

1- To discover a problem. To describe situations.

2- To find out relations.

3- To find out risk factors and causal relationship.

4- To evaluate management methods

Types of the studies

- **Non-intervention studies:**

   - Descriptive studies.

   - Comparative (analytical) studies.

- **Intervention studies:**

   - Experimental studies.

Reference:

What is intervention study?

A prospective study comparing the effect and value of intervention (s) against a control in human being.

Confirm etiological hypothesis & assess effectiveness of preventive measures & new therapies

Reference:

<u>Two approaches</u>

1- Addition of possible causal agent (therapeutic) e.g. testing new drug, implantation of organ.

May be dangerous or fatal for human, for practical and ethical reasons.

2- Protection from causative agent: by removing agent from environment (smoking) or administering a protective measure (preventive) e.g. fluoridation of water supplies or vaccination. These are safe and done on human.

Reference:

Characteristics of experimental study

- **_Manipulation_**: the researcher does something to one group of subjects in the study.

- **_Control:_** the researcher introduces one or more control group(s) to compare with the experimental group.

- **_Randomization:_** the researcher takes care to randomly assign subjects to the control and experimental groups. (Each subject is given an equal chance of being assign in either group)

Reference:

Intervention studies

Investigator determines which individuals are exposed to factor of interest (intervention arm) and which are unexposed (control arm).
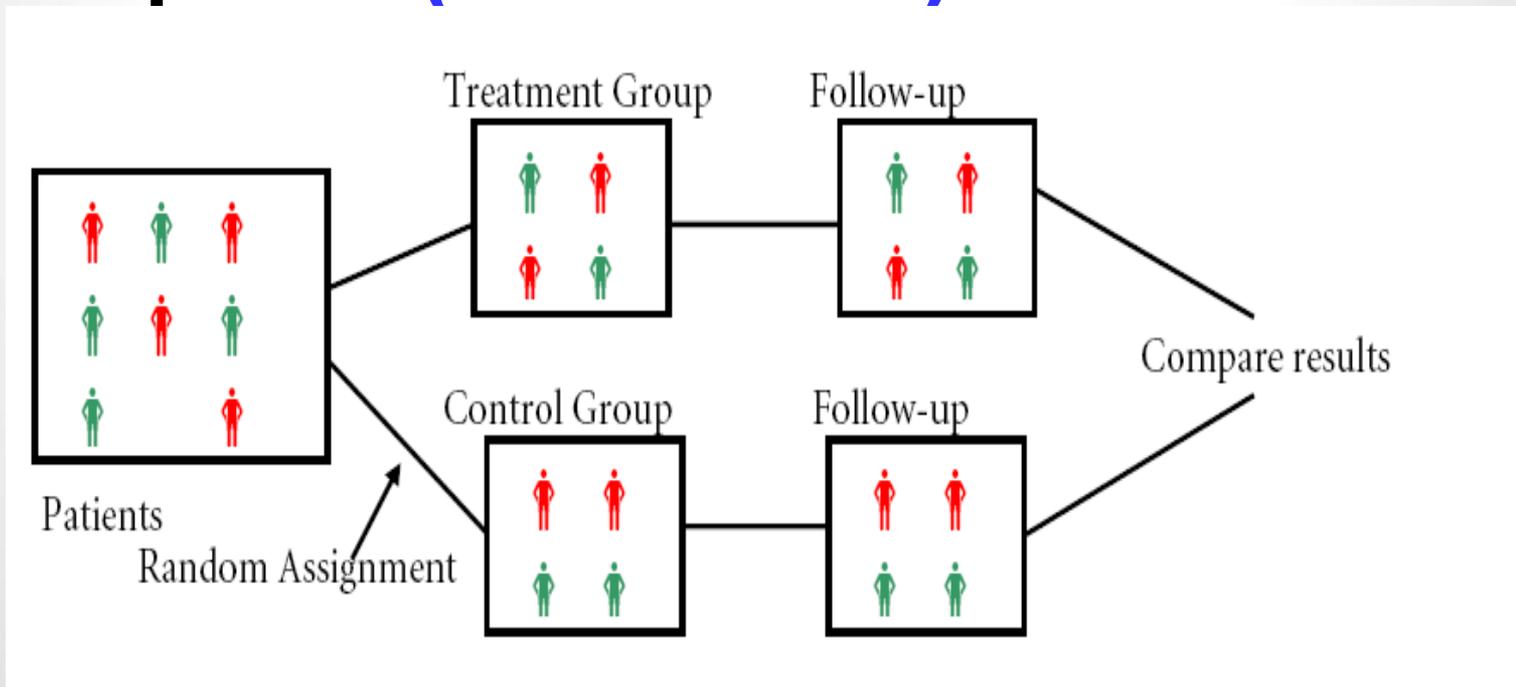
Stages and phases of clinical trials:

Stage 1 (preclinical studies = pre-phase I): involves lab. animals

Stage 2: involves human participants

-Phase I trials

-Phase II trials

-Phase III trials

-Phase IV trials

(post-marketing surveillance)

Reference:

Stage 1 (preclinical studies = phase 0 = pre-phase I) :

In vitro and lab. animals. We look at five things:

- Pharmacokinetics.

- Pharmaco-dynamics.

- Drug metabolism.

- Lethal dose (LD_{50}).

- Teratogenic effects.

Reference:

Stage 2: involves human participants

Phase I trials (20-100 subjects): not randomized, volunteers.

Drugs with serious SE can be tested on seriously ill patients who have failed to respond to current established therapy.

To assess safety, pharmacokinetics, and safe dose range.

Reference:

-<u>Phase II trials</u> (100-200 subjects):

Therapy is still promising after phase I.

Objectives are to set & test dose necessary for pharmaco-dynamic effects, to evaluate potential effectiveness (preliminary efficacy) and to determine optimal method of administration.

Reference:

-Phase III trials (the classical phase) (500-1500 subjects):

- **Randomized double blind controlled trial with adequate sample size & power.**

- **Aim to assess efficacy and additional safety**

- **Used to evaluate whether a new product should be licensed for public use.**

- **Provide decision makers with scientific evidence about relative effectiveness and safety of competing treatments.**

Reference:

Phase IV trials :

Conducted after treatment is approved for general use.

Aim to assess:

*Effectiveness

*Drug safety: long term effects, surveillance for rare SE.

*Drug interactions with other drugs or diets.

*Pharmaco-epidemiology: distribution & determinant of drug use.

*Pharmaco-economics: cost-effectiveness of drug.

*Benefits & harms in presence of comorbidity.

Reference:

Conducting a randomized Clinical trial

A- Formulate a hypothesis.

B- Select participants and get informed

consent.

C- Allocate subjects to comparison groups.

D-Administer treatment and measure outcome.

E. Analyze data

Reference:

C-Allocate subjects to comparison groups

- **By randomization into: study & control groups. (means each subject in reference group has an equal chance to be present in either groups)**

- **Study group exposed to intervention.**

- **Control group: No treatment or Placebo**

- **Both groups must be matched**

Reference:

Blindness

- **Means ensuring that a person "investigator, data collector, or analyst" remains unaware of which arm a subject has been allocated to.**

o **Single-blind:** the subject *participating in the trial.*

o **Double-blind:** *the **subject** & **investigators** (**clinician, interviewers, laboratory personnel**).*

o **Triple blind:** *the **subject**, **investigators** & the **data analysts**.*

Reference:

D-Administer treatment and measure outcome
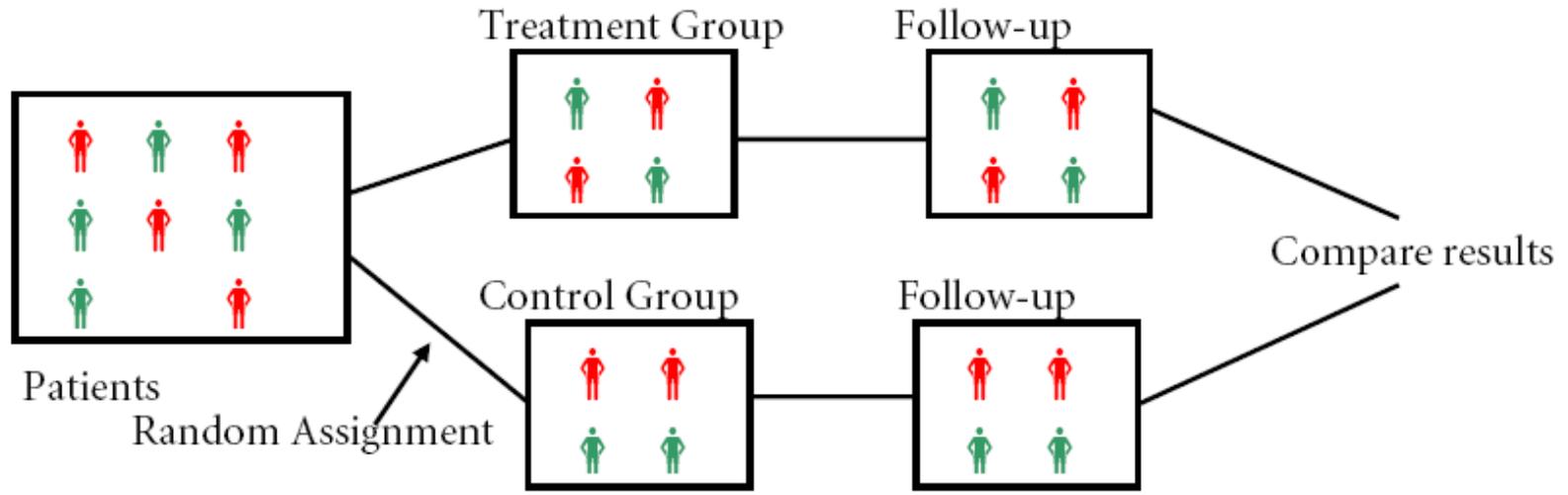
E. Analyze data

- **Compare outcome measures between the groups using statistical tests.**

Summary and rap up

Reference:

Discussion

10 minutes

References

Reference: