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**

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.

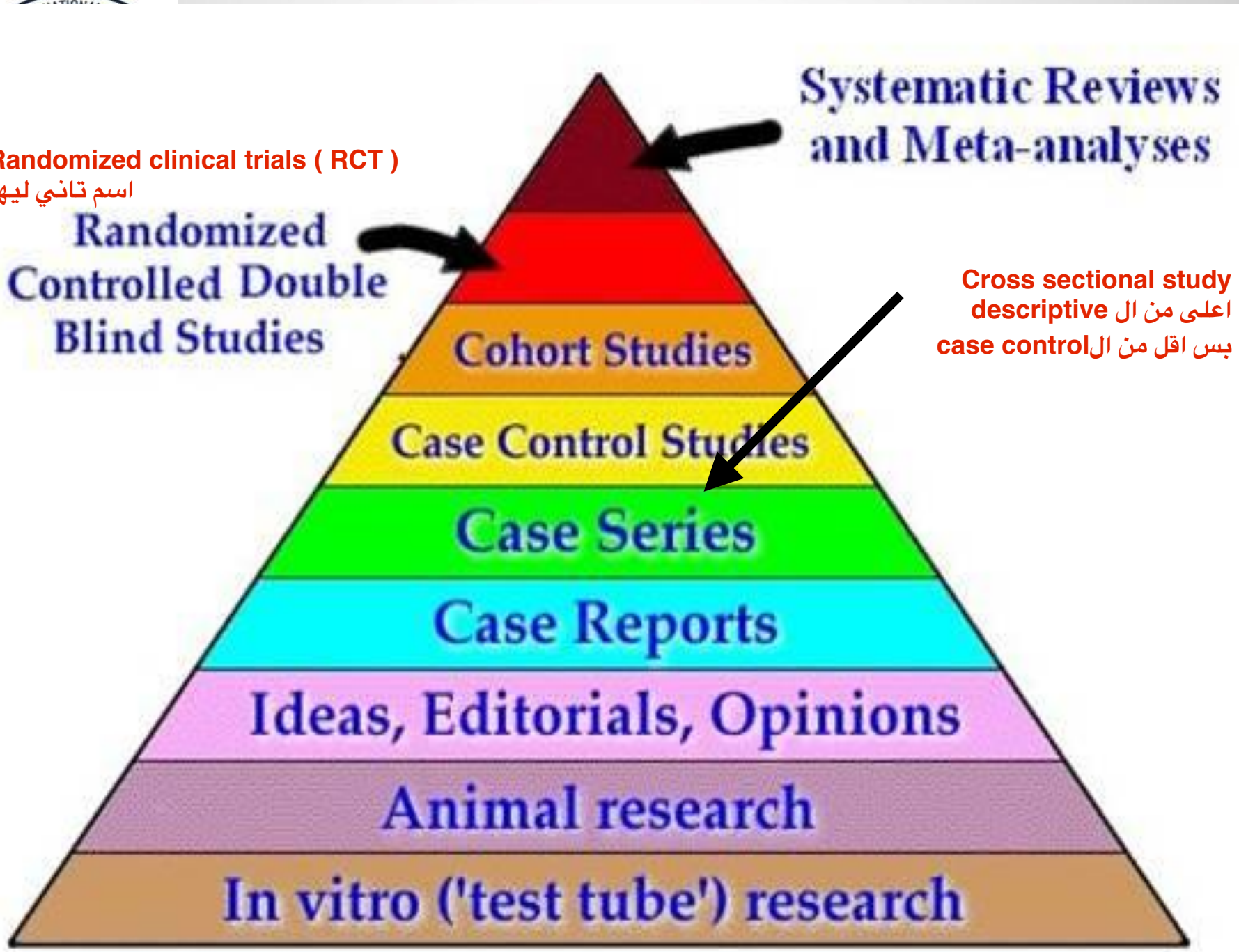
2- To find out relations.

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.**



Randomized clinical trials (RCT)
اسم تاني ليها

Cross sectional study
descriptive اعلى من ال
case control بس اقل من ال

What is intervention study?

Intervention study
clinical trial
experimental study اسامي أخرى لل

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

Two approaches

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.

Characteristics of experimental study

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

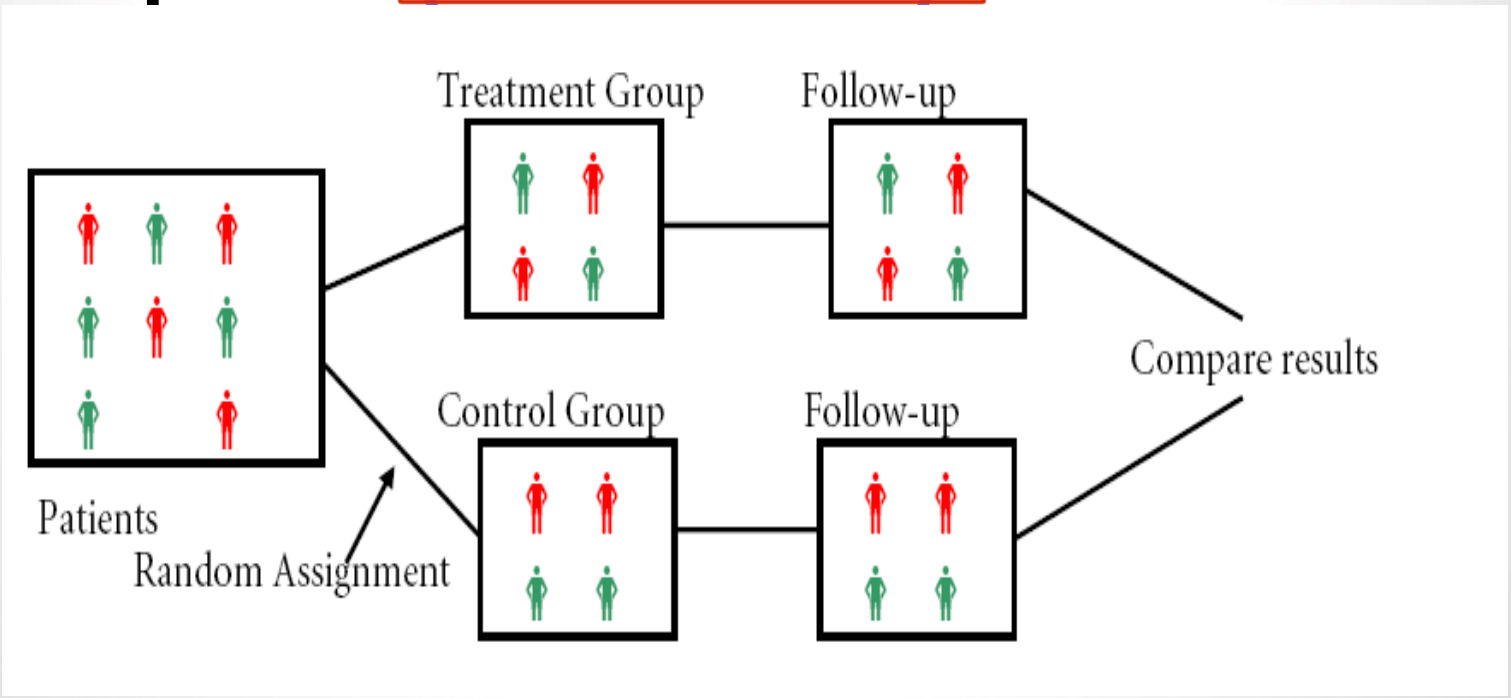
على الاقل مجموعة control واحدة
تأكيد لمعلومة الcase control :
على الاقل control واحد لكل case
وال optimum هو ٢ control وال ideal هو 4

- ***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)

الRandomization اتسألوا فيها
دفعة السنة اللي فاتت (تعريفها)

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)

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}). الكمية اللي بنديها مرة واحدة وتؤدي لموت ٥٠٪ من الحيوانات اللي خدوها
- Teratogenic effects.

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.

-Phase II trials (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.

-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.

Efficacy : results obtained under ideal circumstances
Effectiveness : results obtained under normal circumstances

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.**

هل توزعه بين المدن مختلف ؟

***Pharmaco-economics: cost-effectiveness of drug.**

سعره مقابل كفاءته
مقارنة بالdrugs المنافسة

***Benefits & harms in presence of comorbidity.**

تأثير الdrug في حالة كون المريض مصاب بمرض آخر
مع المرض اللي بنعالجه بالdrug

Reference:

Conducting a randomized Clinical trial

A- Formulate a hypothesis.

B- Select participants and get informed consent.

C- Allocate subjects to comparison groups. مين هياخده

D-Administer treatment and measure outcome. اعطاء الدواء ومراقبة النتائج

E. Analyze data

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

البلاسيبو هو نسخة طبق الأصل من الدواء اللي بنجريه
بس مفهوش المادة الفعالة (يعني ملوش تأثير فعلي)

- Both groups must be matched

ما نسيبش اي عامل يؤثر في النتائج غير الدواء اللي بندرسه
يعني لازم كلهم يكونوا تحت نفس الظروف (
إذا حد عنده سكر يبقى الباقين كلهم سكر برضه وهكذا)

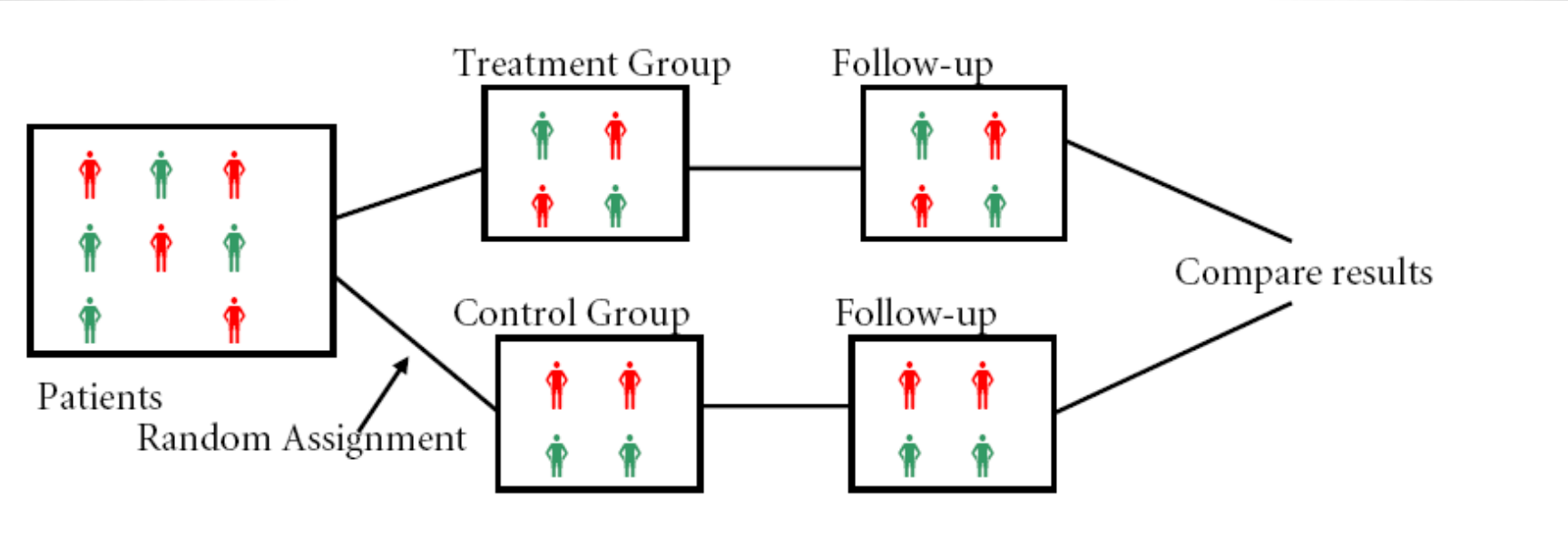
Blindness

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

  - **Single-blind:** the subject *participating in the trial.*
  - **Double-blind:** *the subject & investigators (clinician, interviewers, laboratory personnel).*
  - **Triple blind:** *the subject, investigators & the data analysts.*

ال triple blindness جات قبل كده في امتحان

D-Administer treatment and measure outcome



E. Analyze data

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

The only study that tests causation is ?
Experimental study !

سؤال ذكرته الدكتورة فى آخر المحاضرة ✨

لو وصلت هنا متقفلش لانه في كم معلومة إضافية في السلايد الجاية ✌️

Summary and rap up

معلومة مش في الباور، لو واحد من الcharacteristics مش موجودة
الexperimental study تتحول إلى Quasi study (تحديدا الrandomization)
لو فقدنا الcontrol group بيبقى اسمها pre-post study)
(كلمة Quasi هي الأساس عشان الMCQ، لكن مفيش مانع نعرف الاسم التاني)

معلومة إضافية (الدكتورة ما وضحتش إذا مهمة أو لا بس اعرفها احتياطا)
في حاجة اسمها nested case control وهي كالتالي :
بنبدأ بcross sectional study وطلعنا بprevelance انه فيه ناس مصابة بمرض أيًا كان
فنأخذ الناس المصابة دي ونعاملهم معاملة cases والناس اللي مكانتش مصابة نعاملهم معاملة الcontrol (يعني اتحولوا لدراسة case control)

Discussion

10 minutes

References