Randomized Controlled Trials (RCTs)

Randomized controlled trials (RCTs): brief overview

Suppose we wish to know whether an intervention (new drug, new teaching concept etc) works. The scientific approach to answer this question is to carry out a "trial", involving two groups (e.g. A= Control, B=intervention/treatment).

The distinguishing feature of an RCT is the random assignment of members of the population eligible for treatment to either one or more treatment groups (who receive the intervention / treatment or variations of it) or to the control group (who receive either no intervention).

Random assignment should not be confused with random sampling. Random sampling refers to how a sample is drawn from one or more populations. Random assignment refers to how individuals or groups are assigned to either a treatment group or a control group.

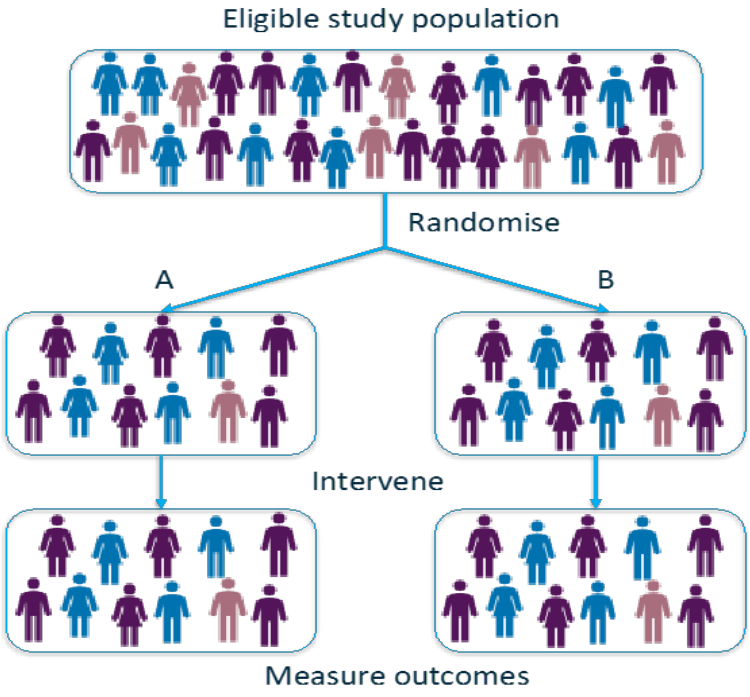

Figure 1: Schematic of the RCT allocation.

Why RCTs?

Through random allocation, the characteristics of participants can be distributed evenly between groups. The RCT design allows us to disentangle 'noise' from what is due to intervention. Random allocation makes it more likely that there will be balancing of baseline systematic differences between intervention groups with regard to known and unknown factors that may affect the outcome (known as selection bias).

Cluster-randomisation

For both practical and ethical reasons, it is more usual to use a cluster RCT design, in which the unit of assignment contains multiple treatment units. For example, education interventions are usually assigned at the school level, although the intervention takes place at the level of the teacher, classroom or individual child, and effects are measured at the level of the child. Nutrition interventions, for example, can be assigned at the community or sub-district level. The figure below shows the allocation of individuals vs clusters.

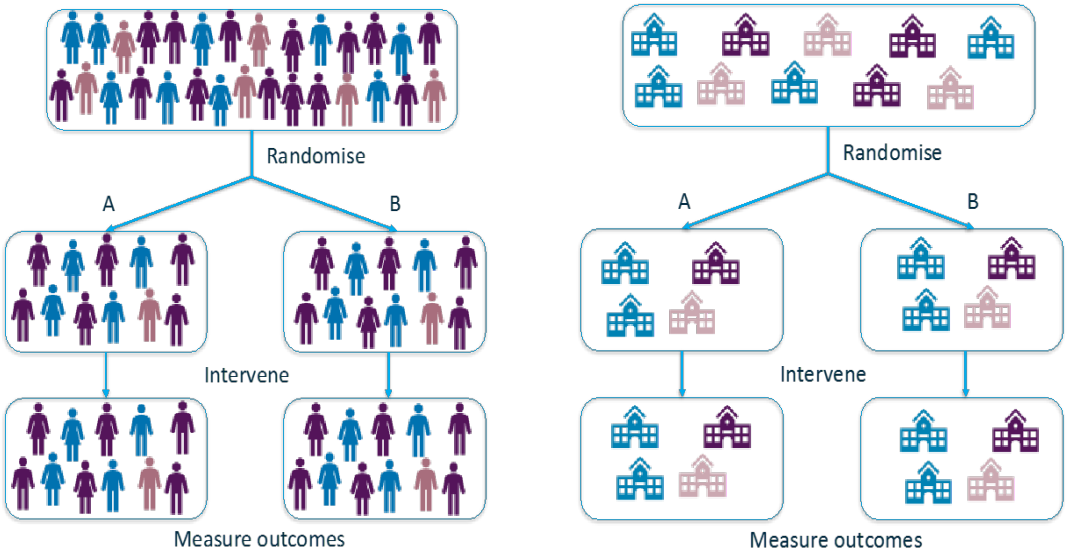

Figure 2: Random allocation of individuals (left) vs clusters (right).

The main advantage of cluster-randomized trials is that they allow for interventions that cannot be directed toward individuals (eg new curriculum, effects of policies). There are some disadvantages, including:

- a larger number of participants is required to achieve the same statistical power (50-200% more),
- recruitment bias (clusters recruited, then randomised, then participants are recruited – allocation is known),
- greater complexity in design and analysis, particularly to deal with the multilevel nature of the data.

Multi-site trials

Cluster-randomized trials should not be confused with multi-site trials (MSTs). For MSTs, the unit of randomization is an individual, rather than a cluster. MSTs randomize treatment assignment separately within each of several sites. The following diagram gives an illustration.

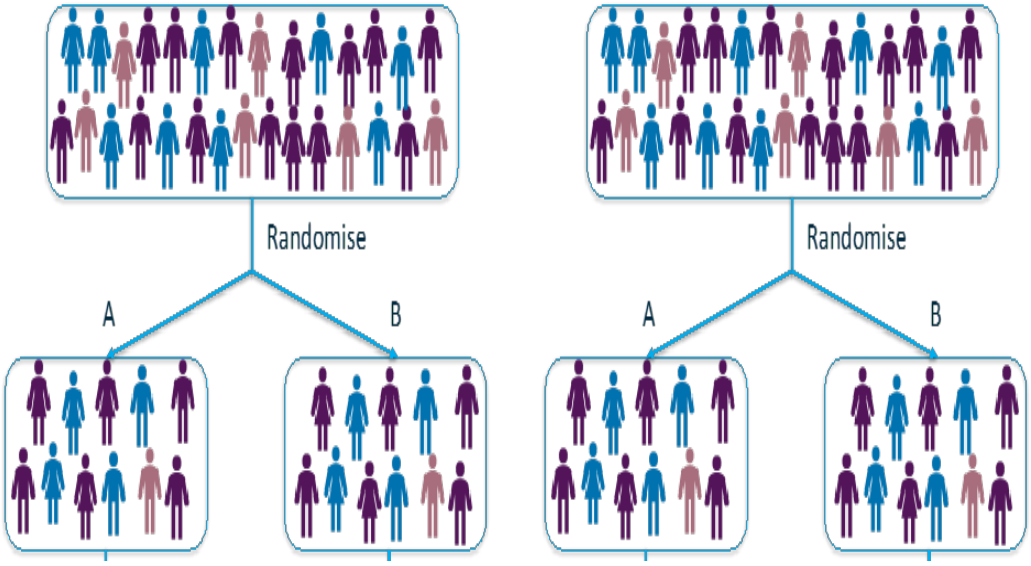

Figure 3: Multi-site randomization. Left (site 1), right (site 2).