

Phases of Clinical Trials

Clinical trials are organised into different categories or phases depending on how much is known about the intervention and what needs to be evaluated.

Phase 0

This level of clinical trial is used to understand the effects of the intervention but does not aim to determine if it has therapeutic effects. Phase 0 trials are conducted in animal models.

Phase I

The first goal is to determine whether the intervention is safe and does not cause harm. Often the participants are healthy volunteers that do not have the condition being targeted. However, for rare diseases and interventions that are high risk such as gene therapy, the participants chosen would be patients with the condition.

Phase II

Once the intervention is deemed to be safe from Phase I trials, the intervention is then tested in the patient population to evaluate efficacy, which means its effectiveness in a small group of patients. Phase II trials will provide preliminary data.

Phase III

If an intervention shows efficacy in Phase II, then its effectiveness will be tested in a larger and more diverse patient population. A Phase III may be where the intervention is compared with a placebo, or a fake intervention. The placebo is used to control for the 'placebo affect', which is where sometimes symptoms can improve slightly without any intervention, simply because the participants are participating in the trial. In order to choose which participants receive the intervention and which receive the placebo, it is often decided at random by a computer. This is called 'randomization'. These kinds of trials are then referred to as "randomized controlled trial' or RCT.

RCTs can be called 'blinded' or 'double-blinded'. This refers to whether those involved in the RCT know which participants are getting the intervention and which are getting the placebo. A 'blinded RCT' means that the participants do not know whether they are getting the intervention or the

placebo, but the clinical researchers on the trial do know. A 'double-blind RCT' refers to trials where neither participants nor clinical researchers know who is getting what. That information is stored within the computer records and is revealed when the data is being analysed.

If an intervention is shown to be safe AND has a measurable effect on the condition that is beyond the placebo effect, the intervention may be approved for use as a treatment in humans by regulatory bodies such as the Food and Drug Authorisation (FDA) in the USA or the European Medicines Agency (EMA).

Phase IV

Once a treatment has been approved and released onto the market, its safety and effectiveness in those who use it continues to be monitored by a regulator such as the EMA.

If you would like to learn about the many different clinical trials that are happening around the world, you can find lots of information at www.ClinicalTrials.gov. This website lists clinical trials in various countries and on numerous conditions. It provides information about the trials. However, it is very important to note that registries such as www.ClinicalTrials.gov do not check the quality of the clinical trial. Registered clinical trials may not have ethical approval, for example, or may not have approval from a national regulatory agency such as the USA's FDA. This means that some of the trials on this site might be ineffective or might pose significant risks. Therefore, it is essential that you discuss your interest in a particular trial with your medical doctor.