

Fact Sheet: Making a medicine – Proof of Concept

Phase II Clinical studies, Proof of Concept studies

Once the volunteer study results have shown that it is safe to proceed, the next step is to start clinical trials in patients with the disease that is being treated. The same guidelines and regulations apply.

In Phase II and Phase III studies, there are usually two treatment groups. One group has the active medicine and one group receives the current best treatment, or a dummy medicine which has no effect on the body (called a 'placebo'). These studies are normally run as 'double-blind', 'randomised', studies.

'Double-blind' means that both the doctor and the participant do not know who is receiving the active medicine or current best treatment/placebo.

'Randomised' means that the treatment groups are chosen by chance. This is usually done with a computer that generates a random code. It cannot be influenced by the doctor or anyone else.

'Placebo-controlled' means that some participants will receive a placebo given under the exact same conditions as the active medicine. This allows the effects related to the medicine to be separated. For example, if a participant in a study complains of a headache it is important to know if that is related to the active medicine. If the same number of participants receiving placebo complain of headaches, this shows that the headache cannot be due only to the active medicine.

All the details of the trial are described in the Study Protocol and the information is collected in the Case Record Form (CRF). The results are analysed using statistical tests.

The more that can be learned about the effect in the patients at this stage, the easier it is to decide if development of the candidate compound should continue. However, Phase II studies are too small to be able to provide sufficient evidence about efficacy and safety. Still, building up more and more information about how the medicine works in patients reduces the risk of failure at the next stage (Phase III or 'Development for Launch'), which is the most complicated and expensive phase of development.

Because these Phase II studies are carried out in patients, the studies are usually run in several hospital sites by hospital doctors – called investigators – as opposed to Phase I studies which are performed in special units. Conducting studies in several different sites at the same time is more complicated than conducting a study in a single site. All of this must be carefully coordinated by the global study team.