Clinical trials - FAQs

At the start of the development of any new drug or vaccine lies an understanding of a disease, the identification of a target (therapeutic or vaccine), and a selection of the most active substances to provide treatment, relief or prevention. It takes two to four years of work to prepare a candidate drug or vaccine for development.

What is a clinical trial?

A clinical trial (also called clinical study) is designed to verify the efficacy and safety of a compound for use by human beings. It takes place after in-vitro and animal studies (pre-clinical testing) have proved satisfactory. The compound, also called the investigational product, is compared either to a placebo (a substance with no pharmacological activity) or to existing treatments, to determine whether it is more or less effective. The clinical trial determines the effective dose regimen, possible toxicity, and the nature and frequency of adverse events it may cause.

Why are clinical trials necessary?

Clinical trials are a mandatory part of the procedure leading to the approval of a drug for marketing. Before new treatments can be made available to all potential patients, they must be deemed effective and well tolerated. Clinical trials are used to validate new treatments and also to define the patient categories for which they are most effective. Finally, clinical trials help to gain a better understanding of the characteristics of a disease.

What are the criteria for the decision to launch a clinical study?

After the identification of a compound under research, it is evaluated in-vitro and then on animals. These steps provide a preliminary assessment of tolerance and therapeutic value. If all the data are satisfactory, the next step is to consider tests involving human beings.

The pharmaceutical manufacturer then submits a request to an Independent Ethics Committee (IEC) or Institutional Review Board (IRB), a completely independent body whose mission is to scrutinize the test procedures and protocol. The committee’s decision is based, in particular, on the pertinence of the project and the protection afforded to participants. The clinical trial may proceed only once it has been approved by the committee, which also follows regularly the progress of the trial to ensure continuity in the safety and welfare of the participants.

What are the risks for patients?

All clinical trials are run according to the principles of Good Clinical Practice (GCP) and are under the supervision of the above-mentioned ethics committees, the mission of which is to ensure the safety and welfare of study participants. Clinical trials can only be conducted if all possible precautions have been taken to protect patient safety. Furthermore, the investigational drug is only tested if there is a real possibility of therapeutic benefit that needs to be evaluated. Participation in a clinical trial may give a patient the opportunity, especially when there are no existing treatments or when they are ineffective or poorly tolerated, to benefit from innovative treatment. Details of the potential adverse events are part of the information given by the physicians to patients invited to participate in a trial. Patients are given time to reflect. In the event that a patient wishes to enter a trial, he or she signs a free and informed consent form stating that relevant information on the study, its procedures, risks and constraints, have been made known to him or her. Biological, medical and clinical supervision of patients throughout the study ensure the highest degree of protection.
Patients may decide, at any time, to withdraw from the trial.

What is the Data Monitoring Committee?

In addition to mandatory safety reporting obligations, requested for the marketing authorization of a product, an independent expert group, or Data Monitoring Committee (DMC), is routinely involved in pivotal clinical trials. The role of the DMC is to ensure complete and independent assessment and surveillance of the safety of all patients participating in the trials. The DMC meets on a regular basis to analyze any safety alerts or signals, and make recommendations for mitigation plans. The committee may even decide to terminate a trial if it deems that trial subjects are exposed to unacceptable safety risks.

Clinical trials: what are the phases?

Clinical trials are conducted by physicians or hospital teams and proceed along three successive phases:

Phase I:

At this point, the compound is tested mainly on a limited number of healthy subjects*, who may receive compensation and are under strict medical supervision. The compound is tested over a short period of time. The purpose is to evaluate the product's safety, how it evolves within the body, the tolerance threshold and adverse events.

Phase II:

Testing involves larger groups of patients. The purpose is to test the product's efficacy and determine optimal dosage regimen. These studies are usually comparative: one of two groups of patients is administered the product whereas the other group is given a placebo.

Phase III:

Testing involves a large number of patients, with the purpose of comparing the therapeutic efficacy of the compound to a reference treatment (if there is one) or to a placebo (when there is no alternative therapy). Such studies are very frequently multi-centric (i.e. involving many study centres). Generally, neither the patient nor the medical profession is aware of what each patient is being treated with (double blind trial). This is to avoid any bias or prejudiced opinion on either side regarding efficacy or adverse events.

* Patients are sometimes recruited to Phase I studies, especially in trials of drugs such as cancer therapies.

Once these three phases have been successfully completed, the resulting data, together with the results of pre-clinical testing, are collected to compose a registration file that will be submitted to public health authorities for license to market.

Phase IV:

Trials do not cease once the pharmaceutical has been put on sale; they continue throughout its marketing life. Trials called Phase IV are carried out after approval in conditions close to those of usual medical care. Specific targets at this point are to detect possible rare undesirable side effects which had escaped attention in the previous phases (pharmacovigilance) and to define conditions of use for certain groups of at-risk patients.

During this phase, drug interactions can be listed and new galenic forms and therapeutic indication extensions can be developed.