

# Clinical Trial Frequently Asked Questions & Answers

## 1. What is a clinical trial?

Clinical trials, also known as clinical studies, test potential treatments in human volunteers to see whether they should be approved for wider use in the general population. A treatment could be a drug, medical device, or biologic, such as a vaccine, blood product, or gene therapy. Potential treatments, however, must first be studied in laboratory animals to determine its safety before they can be tried in people. Treatments having acceptable safety profiles and showing the most promise in the animal model are then moved into clinical trials. Clinical trials are an integral part of new product discovery and development, and are required by all regulatory agencies (e.g. the Food and Drug Administration (FDA) in the United States), before a new product can be brought to the market.

## 2. Why participate in a clinical trial?

The decision to participate in a clinical trial is one that should be made by the patient and his/her loved ones working in close communication with the physician. Participants in clinical trials play a key role in drug development and discovery; clinical trials contribute to knowledge and progress in treating and preventing diseases. First and foremost participants can help others by contributing to medical knowledge and improving public health. Further, a participant does not need to be a patient diagnosed with a specific disease or health problem as some clinical trials, focusing on safety, will include healthy volunteers.

Patients who take part in clinical trials may benefit from the treatments they receive. As part of a clinical trial, a patient will receive either the experimental treatment being tested, an accepted standard treatment for the condition, or a placebo. It is important to understand that there is no guarantee that any treatment received in a clinical trial will produce the desired results.

## 3. What should one expect during a clinical trial?

For all types of trials, participants work with a research or clinical trial team, including doctors, nurses, social workers, and other health care professionals. Prior to the trial, the research team will check the health of the participant and review any special instructions for trial participation. As the trial begins and throughout its duration, the research team will administer treatment, (whether that be the experimental treatment, a standard treatment or a placebo depending on the requirements of the study) and monitor the participant on a regular basis to determine effectiveness and side-effects of the treatment. Ongoing communication is an important part of any clinical trial and after the trial has been completed the research team will stay in touch with the participant for a specified period of time to assess any effects of the treatment after treatment has stopped. The data collected before, during and after the trial is a crucial component to the drug's approval submission to drug regulatory agencies.

## 4. What is informed consent?

Informed consent is the verification of a person's willingness to participate in a research project. Prior to enrollment in a clinical trial, researchers inform participants about all relevant study details and known risks. Participants are then provided an informed consent document that details all the important study information including its purpose, duration, risks, potential benefits, required procedures, and key contacts. Once participants have had a chance to read this form and ask questions, if they agree to participate in the trial, they will be asked to sign an informed consent document. The informed consent document is not a contract.

Participation in the clinical trial is voluntary and the participant may withdraw from the trial at any time without penalty or loss of benefits to which he/she is otherwise entitled. The research team actively maintains informed consent throughout the entire trial by providing the participant with any new or developing information, as needed.

### **5. Who can participate in a clinical trial?**

Before joining a clinical trial, a participant must meet certain criteria. This is an important aspect of any clinical trial to ensure that the treatment is being investigated accurately and safely. Factors that allow someone to participate in a clinical trial are called "inclusion criteria," and those that disallow someone from participating are called "exclusion criteria."

These criteria are used to identify appropriate participants. Acceptance of a participant into a clinical trial is based on such factors as age, gender, the type and stage of disease, previous treatment history, and other medical conditions. For example, some research studies seek participants with specific illnesses or conditions, while others need healthy participants. Some studies may include only men, some studies may include men and women but not women of child-bearing potential, and some studies may include men and women within a specific age range (ie, 18-65 years of age). These criteria are defined by the amount of scientific and safety information that is known about a treatment being tested at the time the trial is planned to start.

### **6. What are the benefits and risks of participating in a clinical trial?**

**Benefits include:** Playing an active role in one's health care, gaining access to medications that may not be available for a significant amount of time, and helping others by participating in the trial so the treatment can potentially be approved and made available to the public.

**Risks include:** Participation in a clinical trial may involve some risks that your doctor will explain in more detail. These risks include:

- side-effects that are known and those that have not yet been identified;
- risks associated with study procedures;
- the experimental treatment may be ineffective or less effective than the current standard;
- the experimental treatment may not work for every patient.

Additionally, in some clinical trials the patient may not receive the experimental treatment, but the current standard or a placebo.

In addition to the risks listed above, the trial might require the participant's time and attention—including trips to the study site, more treatments, hospital stays or complex dosage requirements.

### **7. What should people consider before joining a clinical trial?**

It is important that all prospective participants understand as much as they can about their condition and the clinical trial they are considering. Participants should feel comfortable asking the members of the research team questions about the trial, the care expected while participating, as well as the anticipated cost. Questions participants should discuss with the trial's research team include, but are not limited to, the following:

- What is the purpose of the trial?
- Who is going to be in the trial?
- Why do researchers believe the experimental treatment being tested may be effective? Has it been tested before?
- What kinds of tests and experimental treatments are involved?

- How do the possible risks, side effects, and benefits in the study compare with my current treatment?
- How will my condition and the effectiveness of the treatment be monitored?
- How might this trial affect my daily life?
- How long will the trial last?
- Will hospitalization be required?
- Who will pay for the experimental treatment?
- Will I be reimbursed for other expenses?
- What type of long-term follow up care is part of this study?
- Will results of the trials be provided to me? When?
- Who will be in charge of my care?

### **8. Will I be paid to participate in the clinical trial or is there a cost to participate?**

Compensation for participants is unique with each clinical trial and each sponsoring partner. This is a question that should be discussed with the researcher when considering participating in a clinical trial.

The experimental compound as well as tests and procedures associated with the trial are usually provided at no cost to the participant. In most cases, the cost of routine tests and procedures—not associated with the trial—are the responsibility of the participant, or the participant's insurance carrier.

### **9. What is a protocol?**

A protocol is the study plan on which the clinical trial is based. Each trial is carefully designed to safeguard the health of participants as well as answer specific research questions. The protocol describes in detail what types of people may participate in the trial, the schedule of tests, procedures, medications, dosages, and length of the study.

### **10. What is a placebo?**

A placebo is an inactive pill, liquid or powder that has no treatment value. In a placebo controlled trial, some portion of the participants will receive placebo instead of an active drug or experimental treatment to assess the experimental treatment's effectiveness and safety relative to no treatment at all.

### **11. What is a control or control group?**

A control is the standard by which experimental observations are evaluated. In many clinical trials, one group of patients will be given an experimental drug or treatment, while the control group is given either a standard treatment for the illness or a placebo.

### **12. What are the phases of clinical trials?**

Clinical trials are conducted in a series of stages, called phases, each having specific goals. This process provides information about the treatment in a controlled process intended to also protect the participants. The number of participants in each phase of the trial may be based on the overall incidence of the condition being studied.

Clinical trials are usually classified into one of four phases:

*Phase 1:* Sometimes called dosing, pharmacokinetic, or clinical pharmacology studies, these trials test methods of administering the treatment (e.g. by mouth, injection, etc.) and how often, as well as the safety of the treatment. These trials usually involve a small number of healthy participants (20-80 healthy volunteers).

*Phase 2:* These trials continue to test the safety of the treatment and evaluate how well the treatment is tolerated and how well it works. Phase II studies usually evaluate the treatment in a specific condition. These trials usually involve 100-300 patients.

*Phase 3:* These trials compare the experimental treatment to the current standard of treatment for a specific condition, establishing both efficacy and adverse events. Participants are usually assigned to either receive the experimental treatment or the current standard. Phase III trials typically enroll large numbers of patients (1,000-3,000) and may be carried out at hospitals and doctors' offices nationwide.

*Phase 4:* Post-marketing studies to gain a greater understanding of the treatment, including its risks, benefits, and optimal use. Depending on the purpose of these studies they may be small studies like the Phase I type OR may be even larger than a Phase III study.

### **13. What is a prospective, randomized, double-blind, controlled clinical trial?**

A prospective, randomized, double-blind, controlled clinical trial is the most rigorous clinical trial design, and the one that regulatory agencies mandate must be conducted to demonstrate a medication's effectiveness and safety. In a new drug application, these studies represent the highest quality data regarding the drug and its actions, and form the basis for approval. In this study design, patients are carefully selected for participation and are randomly assigned to receive the experimental drug or a matching active drug or placebo. Neither the patient nor the treating physician knows which treatment was provided, thereby eliminating possible bias. Individual definitions of the study descriptions are:

*Prospective:* Forward looking, beginning before the patient has started treatment.

*Randomized:* Patients are randomly assigned to receive the experimental treatment or alternative (e.g. standard of care or placebo)

*Double-blind:* Neither patients nor the study staff knows which participants are receiving the experimental drug and which are receiving a placebo or standard treatment.

*Controlled:* One group of patients will be given an experimental drug or treatment, while a second group is given either a standard treatment for the illness or a placebo.

### **14. What are side effects and adverse reactions?**

Side effects include any undesired actions or effects of a drug or treatment. Experimental drugs must be evaluated for both immediate and long-term side effects.

### **15. Can a participant leave a clinical trial at any point?**

Yes. A participant can leave a clinical trial at any time. The participant should let the research team know when withdrawing from the trial and the reasons for leaving the study.

### **16. Who sponsors a clinical trial?**

Clinical trials can be sponsored or funded by a variety of organizations or individuals including physicians, medical institutions, foundations, voluntary groups, and pharmaceutical companies, in addition to government agencies such as the National Institutes of Health (NIH), the Department of Defense (DOD), Human Health and Services (HHS), and the Department of Veteran's Affairs (VA).