



A Basic Introduction to Clinical Trials

Choosing to participate in a clinical trial is an important personal decision. It is often helpful to talk to a physician, family members, or friends about deciding to participate in a clinical trial. The following frequently asked questions provide a basic introduction to clinical trials.

What is a clinical trial?

A clinical trial (also called clinical research) is a research study using human volunteers designed to determine the safety and effectiveness of a drug, biologic (such as a vaccine), device (such as a prosthesis) or other treatment or behavioral intervention. Carefully conducted clinical trials are the fastest and safest way to find treatments that work in people and methods to improve health. *Interventional trials* determine whether experimental treatments or new ways of using known therapies are safe and effective under controlled environments. *Observational trials* address health issues in large groups of people or populations in natural settings.

Why participate in a clinical trial?

Participants in clinical trials can play a more active role in their own health care, gain access to new research treatments before they are widely available, and help others by contributing to medical research.

Who can participate in a clinical trial?

All clinical trials have guidelines about who can participate. The 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." Using inclusion and exclusion criteria is an important principle of medical research that helps to produce reliable results. These criteria are based on such factors as age, gender, the type and stage of a disease, previous treatment history, and other medical conditions. Before joining a clinical trial, a participant must qualify for the study. Some research studies seek participants with illnesses or conditions to be studied in the clinical trial, while others need healthy participants. It is important to note that inclusion and exclusion criteria are not used to reject people personally. Instead, the criteria are used to identify appropriate participants and keep them safe. The criteria help ensure that researchers will be able to answer the questions they plan to study.

What happens during a clinical trial?

The clinical trial process depends on the kind of trial being conducted. The clinical trial team includes doctors and nurses as well as other health care professionals. They check the health of the participant at the beginning of the trial, give specific instructions for participating in the trial, monitor the participant carefully during the trial, and stay in touch after the trial is completed.

Some clinical trials involve more tests and doctor visits than the participant would normally have for an illness or condition. For all types of trials, the participant works with a research team. Clinical trial participation is most successful when the protocol is carefully followed, including frequent contact with the research staff. The protocol is a study plan on which all clinical trials are based. The plan is carefully designed to safeguard the health of the participants as well as answer specific research questions. A protocol describes what types of people may participate in the trial; the length of the study; and if applicable, the schedule of tests, procedures, medications, and dosages. Participants in an interventional (treatment) clinical trial are seen regularly by the research staff to monitor their health and to determine the safety and effectiveness of their treatment. Whereas, there are other types of research trials that involve interviews, questionnaires, or surveys instead of interventional treatment.

What is informed consent?

Informed consent is the process of learning the key facts about a clinical trial before deciding whether to participate. It is also a continuing process throughout the study to provide information for participants. To help someone decide whether to participate, the doctors and nurses involved in the trial explain the

details of the study. If the participant's native language is not English, translation assistance can be provided. Then the research team provides an informed consent document that includes details about the study, such as its purpose, duration, required procedures, and key contacts. Risks and potential benefits are explained in the informed consent document. The participant then decides whether to sign the document. Informed consent is not a contract, and the participant may withdraw from the trial at any time. If a participant is a non-English speaking person, federal guidelines require a version of the consent form be provided in a language the participant can understand.

What is a protocol?

A protocol is a study plan on which all clinical trials are based. The plan is carefully designed to safeguard the health of the participants as well as answer specific research questions. A protocol describes what types of people may participate in the trial; the schedule of tests, procedures, medications, and dosages; and the length of the study. While in a clinical trial, participants following a protocol are seen regularly by the research staff to monitor their health and to determine the safety and effectiveness of their treatment.

What is a placebo?

A placebo is an inactive pill, liquid, or powder that has no treatment value. In clinical trials, experimental treatments are often compared with placebos to assess the experimental treatment's effectiveness. In some studies, the participants in the control group will receive a placebo instead of an active drug or experimental treatment.

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.

Do I get to choose which group (experimental or control) to participate in?

No, each person who agrees to participate in a clinical trial that compares an experimental medicine or device with a standard treatment or placebo is randomly assigned (that is by chance) to one or the other group. In general, the participant and the research team do not know the group assignment until after the study is completed.

What difference does it make if I know (or the research team knows) that I am in the experimental or control/placebo group?

Knowledge of this information may influence a participant's or study team's reporting of how things are going in the study. For example, if the participant and/or study team knows that the participant is in the experimental group, an adverse event such as a skin rash might be reported as being "likely" related to the experimental medicine, instead of "possibly related". Or the participant might report adverse events more frequently than if he/she was unaware of the group assignment. However, if the participant and/or study team knows that the participant is in the control/placebo group, the skin rash would be reported as "unrelated" and more importantly, the participant in this group might report worsening of his/her illness or condition, when there has been no change.

What are the benefits and risks of participating in a clinical trial?

Benefits: Well-designed and well-executed clinical trials provide the best approach for eligible participants to:

- Play an active role in their health care decisions.
- Gain access to new research treatments before they are widely available.
- Obtain expert medical care at leading health care facilities during the trial.
- Help others by contributing to medical research.

Risks: Clinical trials entail risks, which may include:

- There may be unacceptable worsening of the illness or condition if the participant is randomly assigned to a control group and/or receives a placebo.
- There may be unpleasant, serious or even life-threatening side effects to experimental treatment.
- The experimental treatment may not be effective for the participant.
- The protocol may require more of their time and attention than would a non-protocol treatment, including trips to the study site, more treatments, hospital stays or complex dosage requirements.

What happens if my illness or condition gets worse while participating in a trial?

A participant has the right to withdraw his/her participation in a clinical trial at any time for any reason including unacceptable worsening of his/her illness or condition regardless of whether or not it is related to the study. It is important for the participant to read the consent form carefully to understand what the consequences (if any) are for early withdrawal from the study.

What should people consider before participating in a trial?

People should know as much as possible about the clinical trial and feel comfortable asking the members of the health care team questions about it, the care expected while in a trial, and the cost of the trial. The following questions might be helpful for the participant to discuss with the health care team. Some answers should be addressed in the informed consent document.

- What is the purpose of the study?
- Who is going to be in the study?
- 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 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?
- How will I know that the experimental treatment is working?
- Will results of the trials be provided to me?
- Who will be in charge of my care?
- What if I change my mind about participating in the study?

Where do the ideas for trials come from?

Ideas for clinical trials usually come from researchers. After researchers test new therapies or procedures in the laboratory and in animal studies, the experimental treatments with the most promising laboratory results are moved into clinical trials. During a trial, more and more information is gained about an experimental treatment, its risks and how well it may or may not work.

What are the different types of clinical trials?

- **Interventional or Treatment trials** test experimental treatments, new combinations of drugs, or new approaches to surgery or radiation therapy.
- **Prevention trials** look for better ways to prevent disease in people who have never had the disease or to prevent a disease from returning. These approaches may include medicines, vitamins, vaccines, minerals, or lifestyle changes.
- **Diagnostic trials** are conducted to find better tests or procedures for diagnosing a particular disease or condition.
- **Screening trials** test the best way to detect certain diseases or health conditions.
- **Quality of Life trials** (or Supportive Care trials) explore ways to improve comfort and the quality of life for individuals with a chronic illness.

For more information about clinical trials in general, and to find clinical trials for individuals with TSC, visit: <http://www.clinicaltrials.gov>.