

What does “single-blind” or “double-blind” mean?

“Blinding” is a procedure in which one or more persons in the research trial are kept unaware of the treatment assignment(s) (control group or investigational group). Single-blind usually means only the researcher knows what treatment or intervention the participant is receiving. A double-blind study means both the researcher (and team) plus the participant is unaware of what treatment or intervention the participant is receiving. This is the most accurate type of study as the risk of conscious and unconscious bias is decreased. The purpose of a “blinded” study design is to remove the unintentional bias that can affect the interpretation of the research information that is collected, if the treatment assignment is known. If the participant’s safety requires it, an independent Data Safety Monitoring Board can rapidly tell the investigator which treatment assignment a participant was given; however, this generally requires the participant to withdraw from further participation in the study.

What are the general 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, which is generally more frequent and thorough than standard medical care.
- Help others by contributing to medical research.

Risks: Clinical trials entail risks, which may include:

- The experimental treatment may not be effective for the participant.
- The study protocol may require more time commitment than standard treatments already available. This may include increased frequency of trips to the study site, hospital stays, missed time from work and time constraints with other family dynamics.
- There may be unpleasant, serious or even life-threatening side effects to experimental treatment.

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

The participant and any guardians or caregivers will be in frequent contact with health care professionals whose first priority is to maintain the health and safety of each participant. A participant or the primary investigator (the doctor you are seeing for this trial) has the right to withdraw his/her participation in a clinical trial at any time or any reason including worsening of his/her illness or condition regardless of whether it is related to the study.

Is an “adverse event” the same as a “side effect”?

No, these two terms do not have the same meaning. An adverse event (or experience) describes an unfavorable event or experience that occurs after a participant begins the research study. The event or experience may be reported by the research participant (such as “I was feeling dizzy all day”) or observed by the researcher (such as an abnormal lab test result). The occurrence of an unfavorable experience or adverse event does not necessarily mean it is associated with (or caused by) the experimental drug, device or treatment. Generally, an adverse event/experience is only considered a “side effect” when, after the study is completed, the event was observed to occur much more frequently in participants who are in the experimental group than in the control group.

Where do I find a clinical trial for tuberous sclerosis complex (TSC)?

For a list of current clinical trials, visit tscalliance.org/clinicaltrials.

Where can I get more general information about clinical trials?

The U.S. National Library of Medicine maintains clinicaltrials.gov, which lists research studies recruiting human volunteers in the United States and more than 200 other countries. The site also provides links to other useful resources, such as:

- A glossary of terms used on the ClinicalTrials.gov website.
- MedlinePlus®, the National Institutes of Health’s website providing information about diseases, conditions, and wellness issues.
- *NIH MedlinePlus Magazine*, which presents up-to-date health information from research supported by the National Institutes of Health.

About the TSC Alliance

The TSC Alliance® is an internationally recognized nonprofit that does everything it takes to improve the lives of people with tuberous sclerosis complex (TSC).

We are a source of hope and connection for all affected by TSC. We drive research, increase care quality, improve access and advocate with and for people affected by the disease. Through our collaboration and partnerships, we’ve advanced FDA-approved treatments and created support systems around the world so no one has to navigate TSC alone.

The TSC community is our strongest ally. With the power of families and the support of donors, volunteers, researchers, educators, industry partners, and more, we can create a future where everyone with TSC can realize their full potential—no matter how complex their journeys are to get there.



Hope no matter how complex™

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A basic introduction to clinical trials



This brochure will answer common questions to help you make an informed decision about participating in a clinical trial.



What is a clinical trial?

A clinical trial is a type of research study using human volunteers designed to determine the safety and effectiveness of a drug, biologic (such as a vaccine), device (such as vagus nerve stimulator) or other treatment or behavioral intervention. Carefully conducted clinical trials are the fastest and safest way to find treatments that aim to improve symptoms or provide better quality of life in people. Interventional trials determine whether experimental treatments or new ways of using known therapies are safe and effective under rigorously controlled conditions. The U.S. Food and Drug Administration (FDA) requires all new treatments to be tested in clinical trials before they may be considered for approval. Observational trials address health issues in large groups of people in natural settings.

What are the phases of clinical trials?

Phase I studies include the initial introduction of new drugs, usually in a small number of healthy human volunteers, over a wide range of doses to determine how the human body processes the drug and whether any adverse effects would preclude testing in patients who might benefit from the new treatment. Often less than 20 volunteers participate in this type of study.

Phase II studies evaluate the effectiveness of a drug or device for a particular symptom or symptoms in patients with the disease or condition under study and carefully monitor for adverse effects. These types of studies generally involve 20 to 400 participants.

Phase III studies are much larger, and often longer, studies conducted after Phase II study results suggest effectiveness of an investigational drug or device. These studies, which generally involve several hundred to thousands of participants, gather additional information about effectiveness and safety. Phase III studies are particularly important for defining the conditions under which the FDA will approve a new treatment to be used.

Phase IV studies are clinical trials that study the side effects over time by a new treatment after marketing has been approved by the FDA and is currently available with a prescription from your provider. The purpose of these trials is to help to obtain additional information about risks, benefits and optimal use of a drug or device.

Where do the ideas for trials originate?

Ideas for clinical trials usually come from researchers working in clinical or academic settings. After researchers test new therapies or procedures in the laboratory and in animal studies (preclinical research), treatments with the most promising laboratory results are moved into clinical trials. During a clinical trial, critical information is gained about a study treatment, its risks and how well it may or may not work to treat a particular indication (or manifestations of a disease). Results of clinical studies often influence the way physicians treat diseases and are sometimes used as evidence by the FDA to approve new treatments or devices for use by health care professionals. Publications, several educational videos and much more.

Why participate in a clinical trial?

Because clinical trials are required for any new therapy prior to FDA approval, major improvements in health care would be impossible without volunteer participants. Participants in clinical trials can play a more active role in their own health care, gain access to new investigational 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 and criteria regarding who can participate. The factors that allow someone to participate in a clinical trial will vary from study to study. These guidelines and criteria are determined based on the study's goals and include such factors as age, the type and stage of a disease, previous treatment history and other medical conditions.

Some research studies seek participants with illnesses or conditions to be studied in the clinical trial, while others need participants without underlying health conditions. The criteria are used to identify appropriate participants needed to answer the scientific questions being asked while keeping them safe.

What happens during a clinical trial?

The clinical trial study team, which includes doctors, nurses and other health care professionals, checks the health of the participant at the beginning of the trial, gives specific instructions for participating in the trial, monitors the participant carefully during the trial and stays 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. Clinical trial participation is most successful when the study protocol is carefully followed, including frequent contact with the clinical trial team.

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 all aspects of the informed consent form, the type of health care expected while in a trial and how the trial is intended to help improve health care. You may also discuss key logistics around what is required to participate in a study, including duration, types and frequency of appointments, location, transportation, costs that may not be covered by the trial organizers and other details. These issues are important to discuss because adherence to the clinical protocol is crucial for data collection within the study to advance potential therapies.

What is informed consent?

To help an individual or family make a fully informed decision on whether to participate in a clinical trial, doctors and nurses involved in the trial will share details about their research, including goals of the study, all required procedures and any potential risks and benefits to the potential participant. If the potential participant chooses to participate in the study, he/she or a parent/guardian will sign the informed consent form to document the participant or parent/guardian has been fully informed about the study and that the participant consents to be enrolled in the study. Informed consent is not a contract, and the participant may withdraw from the trial at any time for any reason.

What is a clinical protocol?

A protocol is a study plan on which each clinical trial is based. The plan is carefully designed to safeguard the health of the participants as well as to answer specific research questions. A protocol describes the criteria for participation in the clinical trial; the schedule of tests, procedures, medications and dosages; and the length of the study.



What is a control or control group?

A control group is the standard by which study observations are evaluated. In many clinical trials, one group of study participants will be given an investigational (also called "study") drug or treatment, while the control group is given either a standard treatment for the illness or a placebo.

What is a placebo?

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

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 a study drug or device with a standard treatment or placebo is randomly assigned (that is, by chance) to one of the groups. In general, the participant and the clinical trial team do not know the group assignment until after the study is completed. This approach enables unbiased data collection and reporting from participants and clinical study leaders.

What difference does it make if I know (or the clinical trial team knows) 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 the participant is in the study group, an adverse event such as a skin rash might be reported as being "likely" related to the study drug, 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 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 unintentionally report worsening of his/her illness or condition, when there has been no change.