

An Introduction to Clinical Trials



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What is a clinical trial?

A clinical trial is a medical research study that finds ways to more effectively prevent, diagnose or treat diseases in humans. Clinical trials measure the safety and effectiveness of new treatments, like a new medication or a new use of an existing medication; medical devices (such as pacemakers); or tests and procedures for diagnosing illnesses. To be considered for a clinical trial, participants are prospectively assigned the experimental treatment (and in some cases placebo), to measure the effect of the experimental treatment on some aspect of human health.

Before an experimental treatment, device or procedure can be tested in a clinical trial, it must have shown benefit in laboratory testing, animal research studies or research in a small group of humans. Clinical trials must follow the same (and often stricter) ethical and legal guidelines as standard medical practice to protect the safety of participants. Those laws are made to make sure that the studies do not cause harm to people.

Clinical trials are done in phases. Each phase of the clinical trial has a different purpose.

- **PHASE 1**

- Experimental treatment is given to a small group (20 to 80 people) for the first time to evaluate its safety, dosage range and side effects.

- **PHASE 2**

- Experimental treatment is given to a larger group (100 to 300 people) to evaluate its safety and effectiveness.

- **PHASE 3**

- Experimental treatment is given to large groups (1,000 to 3,000 people) to confirm its effectiveness, monitor side effects and compare to current standard of care treatments or placebo.



If the experimental treatment works well in a Phase 3 trial, researchers can apply to the U.S. Food and Drug Administration (FDA) to ask for permission to make the treatment available to the public. The FDA approval process generally takes about a year. The FDA's review process is carried out in different stages to review the clinical trial's research and data and to decide if it can be a helpful treatment.

In some cases, research continues even after the FDA has approved a treatment. The FDA can ask researchers to conduct a Phase 4 trial.

■ PHASE 4

Researchers collect all the information they have on the experimental treatment's long-term safety and effectiveness after the drug is approved.

Should you consider participating in a clinical trial?

The decision to participate in a clinical trial should involve talking to your doctor, your family and caregivers, the clinical trial team and sometimes your insurance company to see if it is an option for you.

“My doctor asked me to consider participating in a clinical trial...the trial was carefully explained and the process outlined. Before being accepted into the program, I underwent a few months of testing which continued at regular intervals once the trial drug was being administered. Between doctor’s visits, lab testing, and the availability of the clinical coordinator for questions, I always felt safe. Maybe one day this will be the treatment of choice, made possible by all of us participating in this clinical trial.”

Connie

Potential benefits of clinical trials include:

- Access to new research treatments
- Access to specialists and/or expert care
- Contributing to medical research that can help others

Potential risks associated with clinical trials include:

- Serious side effects
- Unsuccessful treatment outcome
- Inability to participate in other trials

What do I need to know about insurance coverage and clinical trials?

Many states have laws or agreements requiring health insurance plans to cover at least the cost of routine care when participating in clinical trials. However, health insurance coverage of clinical trials may be different depending on the company, the plan and the location of the

clinical trial. Health insurance companies or plans may cover all patient costs associated with the clinical trials, while others may cover some or none of the costs.

You should check with your insurance company before you sign up for a clinical trial so that you will know what is or is not covered ahead of time. Call your company's Member Benefits number, which can be found on the back of your insurance card. Give them the clinical trial information, and they will be able to tell you whether the costs will be covered in full, in part or not at all.

Another important person to talk with is the clinical trial coordinator, whose role includes providing important information to the people who take part in these studies. The clinical trial coordinator can tell you about all the patient care costs associated with the clinical trial. The contact information for the clinical trial coordinator should be listed on every trial.

Who can take part in a clinical trial?

Every clinical trial has guidelines and requirements about who can participate, depending on the goals of each trial.

Different trials carry out studies on people from all backgrounds. Some clinical trials may be looking at how different tests work on people who have certain illnesses. Some may look only for people of a certain age, gender, race, ethnicity, or with a specific disease, stage of disease or treatment history. Others may be looking for people without serious health conditions.

What information will you receive before you join a clinical trial?

If you qualify for a clinical trial, you will speak with the clinical trial team before you begin

the trial. The clinical trial team is made up of doctors, nurses, social workers and other healthcare professionals. They will review the clinical trial protocol with you. The clinical trial protocol will describe the goals of the study and how the study will be designed and carried out. The clinical trial protocol includes but is not limited to:

- Clinical trial process, including tests that may be conducted
- Known risks and benefits of experimental treatment
- Length of clinical trial
- Clinical trial contact information
- Contact information for the team who is doing the research

The **Informed Consent** process includes providing the above details, as well as explaining your rights as a research participant. If after receiving all this information you fully understand the study and wish to voluntarily participate in the clinical trial, you will be asked to sign an **Informed Consent** form.

You may choose to stop participating in a clinical trial at any time, even if you have signed the Informed Consent form. You should keep a copy of your Informed Consent form, whether you stay in the trial or choose to leave.

What should you expect during the clinical trial?

Generally, at the beginning of the clinical trial, each participant's health is checked, and the team provides instructions to the participants. The team will then monitor you and the other participants closely during the clinical trial and will follow up with everyone after the clinical trial is completed.

“I learned about the clinical trial through the Wilson Disease webpage. I found it great that this trial could help the present and future Wilson patients. Since I agreed with the purpose, and all my concerns and questions were clearly answered, I signed on. Clinical trials are important, and everyone eligible should consider participating because the value is huge for the person and the community.”

Eleni

It should be made clear to you what is being measured, but you may not necessarily be told why they are requesting it. Measurable testing can include checking blood pressure, heart rate, or blood test results and X-rays and sometimes tissue specimens (e.g. liver biopsies). These tests may be repeated often to make sure the results are accurate.

Where can you find information about current clinical trials?

For information on clinical trials in your area or across the nation:

- Through your medical provider or local hospitals
- Antidote Clinical Trial Finder (www.antidote.me/)
- National Institute of Health Clinical Trials (www.nih.gov/health-information/nih-clinical-research-trials-you)
- Medline Plus (www.medlineplus.gov/clinicaltrials.html)

“The opportunity to participate in a research trial made me feel like I was not just a patient dealing with illness, but that I was also helping to find treatment or a cure. It is an empowering feeling to know that by being in a trial I am helping future patients. The trial I was involved in was very well organized, and I had specific contact people that were very quick to reply to my questions and concerns. It was so nice to have someone who knew my entire health history and was familiar with my disease.”

Jess

What are the next steps if you are interested in a clinical trial?

Speak to your doctor for his or her input. When you are ready to find out more information about the clinical trial, reach out to the clinical trial’s coordinator to see if you meet the requirements. Their information is public and should be included in any announcement about the trial.

If you meet the initial requirements, you will be scheduled for a pre-trial screening during which tests will be done to help researchers decide if you are a candidate for the trial. The pre-trial screening will also be an opportunity for you to learn more about the clinical trial, including its benefits and risks. Screening can include a combination of phone interviews, online questionnaires and in-person testing.

Will the clinical trial's results be posted?

Once data is finalized, it will be published and linked to the clinical trial's page. It will be found on the tab named, "Results Posted". If results are not yet available, the tab will be named, "No Results Posted".

Questions to Ask Your Healthcare Provider

- Are clinical trials a choice for me and if so, which types?
- Do you know of any clinical trials that might be a good choice for me?
- What are the risks and benefits of taking part in a clinical trial?
- If I am assigned to a placebo group, will the treatment be available to me after the trial?
- How will I know if I am having positive or negative side effects from the experimental treatment?
- How will taking part in a clinical trial affect my other health conditions?
- Can I continue to take my usual medications if I participate in the clinical trial?
- Do I follow up with you or the clinical trial team during the trial?

Questions to Ask the Clinical Trial Team

- What is the purpose of this clinical trial?
- Why is this experimental treatment believed to be effective?
- What are the risks and benefits associated with this clinical trial?
- How do the possible benefits and risks of this trial compare with my current treatment?

- What short and long-term effects will this trial have on my daily activities?
- What is expected of me if I take part in this clinical trial?
- What kinds of tests are involved?
- How long will the clinical trial last?
- Is it possible that I may receive a placebo?
- Will I need to pay for any part of this clinical trial?
- How will I know that the experimental treatment is working?
- What happens if my condition gets worse during the clinical trial?
- Whom do I contact if I need to leave the clinical trial, and what is the best way to reach that person?
- Who will be responsible for my care?
- Should I continue to see my own doctor during the clinical trial?
- What type of long-term follow-up care is part of this study?
- What happens at the end of the clinical trial?
- Will I be told the results of the clinical trial? When?

Glossary

Clinical trial: A clinical trial is a research study that is done to find out if a test or treatment given to people is safe and effective before it is made available to the public.

Control group: A control group is made up of people who receive either the treatment that is currently used, or a placebo. The control group serves as a comparison group to measure the effectiveness of the experimental treatment other participants are receiving.

Double-blind study: A double-blind study is a clinical trial in which neither the participant nor the researcher knows which treatment the participant is receiving until the clinical trial is over.

U.S. Food and Drug Administration (FDA): The Food and Drug Administration (FDA) is a government agency responsible for making sure that all medications, vaccines and medical equipment are safe and effective.

Informed consent: Informed consent is the process of learning all details about the clinical trial before you decide to participate.

Institutional Review Board (IRB): An Institutional Review Board (IRB) is a committee of health care professionals and community members who review, approve and monitor clinical trials to make sure potential risks are as low as possible and that the clinical trial follows ethical and legal codes for medical practice.

Placebo: A placebo is an inactive pill, liquid or powder that looks like the experimental treatment but has no effect on the body. In some clinical trials, experimental treatments are compared with placebos to evaluate the effectiveness of the experimental treatment.

Protocol: A protocol is the clinical trial plan that explains the purpose and process of the trial. A protocol will include information such as who can participate; how many people will participate; what the treatment plan involves; type and frequency of tests; how the results will be measured; reasons why the clinical trial may be stopped; reasons why the researchers may stop giving the experimental treatment to a participant; known and likely side effects of the experimental treatment; and possible benefits of the experimental treatment.

Randomization: The selection process by which all study participants have an equal chance of being selected for the treatment or control group.

Single-blind study: A single-blind study is a type of clinical trial in which only the researcher knows which treatment (experimental treatments versus current standard or placebo) the participant is taking until the trial is over.

Treatment group: The treatment group receives the vaccine or medication that the researchers are testing before it is approved by the FDA.



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