



Frequently Asked Questions About Clinical Trials

Opportunities to help develop new drugs from cystic fibrosis research have never been more promising. More potential therapies are in the CF drug development pipeline today than in the entire history of cystic fibrosis research. While that gives all of us great cause for hope, it also charges us with recruiting more people than ever before to help us test new drugs. That's the only way we'll be able to get them to the children and adults who desperately need them. **Participants in clinical trials are the key to new treatments and a cure.** Research cannot move forward without them.

What happens during a clinical trial?

At the beginning of a trial, the research team—including doctors, nurses, and other health care professionals—checks the person's health and tells them everything they need to know about participating. The health check may involve a physical exam and screening tests.

People found eligible for the trial and agree to participate are asked to sign an Informed Consent Form to become enrolled. The research team continues to check the participant's health carefully during the trial and for a period of time after the trial is over.

Some clinical trials require more tests and doctors visits than usual. It is important to the success of the trial that participants do everything expected of them and stay in close touch with the research team.

Who can participate?

All clinical trials have guidelines about who can join. Some enroll healthy people. Others enroll only people with certain conditions such as CF. These guidelines ensure the findings will be accurate and useful. If a trial's guidelines match your condition, you may be eligible to join.

To find the right type of people to enroll in research and to keep them safe, researchers must look at reasons for including and excluding certain people from trials. Inclusion criteria are traits everyone must have to be in a certain trial.

For example, a trial for a new CF medication would require that all participants have a confirmed diagnosis of CF. Exclusion criteria are traits people cannot have in order to be in a certain trial. For instance, a trial for medication to clear mucus from the airways of young children would exclude adults.

Will my CF care team stay involved in my care?

Your CF care team's involvement will depend on the requirements of the trial. We encourage you to discuss trial participation with your CF care team.

What do words like protocol, placebo, randomized and control group mean?

A protocol is a detailed plan for a clinical trial. Aimed at answering certain research questions, the plan is carefully put together to protect the health of participants.

A protocol outlines:

- The goal of the research;
- What types of people may participate;
- The schedule of tests, procedures, medications, and dosages; and
- The length of the trial.

A placebo is an inactive medication that will not affect a person in any way. It is no more than a sugar pill. In clinical trials, people taking experimental drugs are often compared with those taking placebo drugs to learn how well a drug really works.

When a patient is randomized, it means that he/she is assigned by chance to one of the treatment groups. This helps make sure that each study participant has an equal chance of receiving the study drug or the placebo.

In many clinical trials, one group of patients is given the experimental drug or treatment, while the control group is given standard treatment or a placebo.

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These and many other commonly used research terms are defined in the Glossary section.

What are the different phases of clinical trials?

The process of drug development begins in the laboratory. New approaches to treatment are tested for several years—first in the test tube, then in living systems or models. These early tests are called “preclinical” research. If this research proves promising, researchers will ask for approval from the Food and Drug Administration (FDA) to begin clinical trials with patients.

Clinical trials with patients are done in phases. Trials at each phase are done for different reasons and help scientists answer different questions.

In **Phase 1 trials**, researchers test an experimental drug or treatment in a small group of people to 1) learn if it is safe, 2) find a safe and tolerable dosage range, 3) decide how to administer the drug (i.e., orally, intravenously, inhaled, etc.) and 4) learn the side effects.

In **Phase 2 trials**, the experimental drug or treatment is given to a larger group of people to see how well it works (i.e., efficacy) and to keep testing its safety. At this stage, the trials do not provide enough information to know whether or not a drug works to treat an illness, but may people may report some benefits.

In **Phase 3 trials**, the experimental drug or treatment is given to large groups of people to 1) continue testing how well it works, 2) determine dosage amounts, 3) watch for side effects, 3) compare it with commonly used treatments, and 4) collect information regarding its safety. The drug is usually put on the market after Phase 3 trials are completed and the FDA approves the drug.

Phase 4 trials provide more details about a drug, including how well it works over a long period and how it affects people’s quality of life. They also look at how much a drug costs compared with how well it works and may compare that with other new and standard treatments.

What are some of the benefits and risks of participating in clinical trials?

There are both possible benefits and risks to participating in clinical trials.

Possible benefits include:

- Taking an active role in your own health care;
- Gaining access to new treatments not available to the public;

- Getting expert medical care at leading health care facilities;
- Helping others by contributing to medical research; and
- Receiving a treatment that works for you.

Possible risks include:

- Side effects or adverse reactions to medications or treatments;
- Receiving a treatment that doesn’t work for you; and
- Being required to travel to the study site, receive treatments, stay in the hospital, or follow complex dosage requirements.

Can I leave a clinical trial after I have joined?

Yes, you can leave a clinical trial at any time. If you choose to leave or withdraw from a trial, you should let the research team know. They may ask you to come for a final visit.

How safe are CF clinical trials?

Nothing is more important than safety in developing new CF treatments. There are four layers of protection in every CF clinical trial.

Each trial must be determined as safe and appropriate for patients by the:

1. CF Foundation
2. U.S. Food and Drug Administration (FDA);
3. Participating hospital or university’s Institutional Review Board (IRB); and
4. Data Safety Monitoring Board (DSMB). The DSMB is an independent committee of experts in CF care that checks information on ongoing trials, watching for possible problems or unwanted side effects.

The CF Foundation is the only voluntary health organization to organize a DSMB whose members are experts in CF and completely independent and not involved in any way with the trial or its participants. In this way, the CF Foundation does its best to keep participants safe throughout the clinical trial.

How is my safety protected broadly?

The U.S. government has strict guidelines and safeguards to help protect people who choose to participate in clinical research. Every clinical trial in the United States must be

approved and monitored by an IRB. This is to keep risks as low as possible and ensure that the risks are worth any potential benefits.

The IRB is usually made up of doctors and the general public. They look at the trial's protocol (a clear and detailed plan of the experiment) to make sure that participants' rights are protected and the trial does not cause them unnecessary risk.

The FDA also must approve all clinical trial protocols and make sure all of their procedures are being followed as the trial goes on.

In the United States, anyone participating in a clinical trial must sign an Informed Consent Form. This form explains the trial in full, including the risks, and a research team member will explain the trial and the consent form to individuals before they sign it.

How long does each phase of a clinical trial last and what are the associated risks?

It usually takes an average of five to 10 years to take a new drug from the start of a clinical trial to the day it's approved. The CF Foundation has been able to shorten that time.

Generally speaking:

- Phase 1 clinical trials take several months to a year.
- Phase 2 trials take up to 2 years.
- Phase 3 trials usually last several years.
- The FDA may take several months or longer to approve the drug.

Each phase carries a risk that the drug might not work or may have unwanted side effects. Only one in five drugs tested on humans ever becomes available to the public.

How do you choose which drugs will be tested in clinical trials?

Because only one in five drugs tested ever becomes available to the public, it's important to support many different trials at the same time. And, with both money and participants for clinical trials in short supply, it is important to choose those drugs carefully.

The CF Foundation and CFFT have top-notch independent CF researchers on their boards who look closely at all of the available information and recommend which drugs to study. These trials are then made public for people to learn more about them.

At what age can someone join a clinical trial?

The age that someone with CF can participate varies according to each trial. The age range depends on a number of factors, with safety always first in mind.

In clinical trials where participants take medications, drugs must be shown to be safe and work in older patients before they can be tested in young children. Also, young children metabolize drugs differently than adults, so it is important to test drugs in children as well as adults.

How can I learn about CF clinical trials in my area?

CF clinical trials are generally offered at accredited CF Foundation care centers. The first place to check is with your CF care physician. You also may want to check the Find A Clinical Trial section on this Web site, or call our toll-free Clinical Trials Hotline at 1-877-8CF-JOIN (1-877-823-5646). The National Institutes of Health also publishes a list of clinical trials at www.clinicaltrials.gov.

Who at my CF care center should I ask about clinical trials?

You can ask your CF physician or member of the health care team about clinical trials. Also, you may ask to talk with the CF research coordinator at your care center who can answer questions about studies and trials.

What questions should I ask about participating in a clinical trial?

When you speak with your CF physician or the research coordinator at your care center, consider asking the following questions:

- What is the purpose of the study?
- What will be asked of me?
- What will be my role in the study: healthy volunteer or participant?
- Who will be in charge of my care?
- Will the study benefit me?
- Will the study benefit others?
- Why do researchers think that this particular drug or treatment might work?
- What kinds of tests and experimental treatments are involved?

- How do the possible risks, side effects and benefits compare with my current treatment?
- How might this study affect my daily life?
- How long will the study last?
- Will hospitalization be necessary?
- Who will pay for my participation in the study?
- Will I be reimbursed for any expenses?
- How will I know if the experimental drug is working?
- Will results of the studies be given to me?
- What type of long-term follow-up care will be required?

Will I get early access to drugs if I participate in clinical trials?

Trial participants—even those who received a placebo at first—sometimes get to take the test drug for a period of time. Ask the trial’s contact person to see if this is the case in the trial you’re considering.

What does “expanded access” mean?

In a clinical trial there are certain requirements that a patient must meet to be able to participate. For instance, in a trial for CF, a patient may need to have to a minimum lung function. If they don’t meet the requirements, they would not be able to participate in the trial.

For patients who may benefit from a potential drug but don’t qualify to participate in a trial, FDA regulations enable manufacturers of investigational new drugs to provide for “expanded access” use of the drug. The primary intent of the expanded access program is to allow access to a new drug for people with a life-threatening or serious disease for which there is no good alternative treatment.

The secondary purpose of expanded access is to generate additional information about the drug, especially its safety. Expanded access protocols can be undertaken only if clinical investigators are actively studying the experimental treatment in well-controlled studies, or all studies have been completed.

To find out more about expanded access, visit www.clinicaltrials.gov.

I have participated in trials and have never heard about the results. Why?

It’s very important to communicate results with people who participate in trials. However, there are several reasons why trial results may be released either very slowly or not at all.

- The CF Foundation must coordinate with a number of drug companies and universities to run the trials.
- The trials themselves can take a long time. Participants may be recruited over several years and getting the results back after the final volunteer has been tested can take a long time.
- It may take a while to analyze the results of the trial to find out if it helps people with CF.
- Only one in three drugs ever makes it to Phase 3 of a clinical trial. Results for drugs that do not make it are never published. When results can be published, a strict review process slows down the reporting. Publication may not happen for a year or more after the trial is over.

All of these factors make it difficult to communicate the results to the CF community. Even so, the CF Foundation is committed to improving communication of results to trial participants.

Who decides how much money clinical trial participants are paid?

How much money is paid to participants, if anything, is up to the trial sponsor. The amount of payment often depends on the kind of trial and what participants will be asked to do. Some researchers want to pay more so that that it does not take so long to recruit participants. Others feel that money should not be offered.

Participants in CF clinical trials are usually reimbursed for travel and meals.

Please note, because accepting payments for participation may affect your continued eligibility for Supplemental Security Income (SSI), Medicaid or Medicare low-income subsidies, please talk to your research coordinator before participating in a clinical trial that offers payment for participation.

The CF Foundation is working to resolve this issue and will inform you of progress. You can help reverse this policy and enable more people to participate in CF clinical trials by contacting your Representative to ask him or her to support “The Improving Access to Clinical Trials Act,” legislation to remove this barrier.

Are there plans to collect and compare clinical trial information from outside the United States?

The U.S. CF Foundation has close ties to CF groups in Europe, United Kingdom and Australia. We provide research support and chances to work with U.S. scientists. Also, the CF Foundation is working with more than 30 European Union members to create a global CF Patient Registry. It will make international research easier and help us identify the best patterns of CF care. This global registry will have the same standards of confidentiality as we use here in the United States.

Why aren't all CF care centers involved in clinical trials?

Larger CF care centers have more people with CF and can more easily recruit for clinical trials. These centers have clinical research coordinators who recruit people with CF and also help conduct trials. However, the CF Foundation is working hard to give more people everywhere the chance to participate in trials.

Our Web site has information on what everyone can do to participate. A Clinical Trials Hotline (1-877-8CF-JOIN) also is available to help answer questions. Finally, the CF Foundation has increased the number of care centers who have research coordinators and can offer clinical trials.

What can I do to help bring CF treatments to the CF community sooner?

The single most important thing you can do is participate in clinical trials. Find out what trials are recruiting at your center. Visit our Find A Clinical Trial section to look for available trials. CF researchers are working hard to find new ways to treat CF, but not a single new treatment can be tested without including people with CF.

What does the future look like for my child with CF? Is a cure in sight?

No one knows when the cure for CF will come, but the outlook for children born today with CF has never been brighter. New treatments are now being developed that should make a real difference in the way lung disease and nutrition issues are managed. Today, young children with CF have an excellent chance to live a full and rewarding life.

Other than raising enough money for research, what are the biggest challenges to finding a cure for CF?

Finding enough people to participate in clinical trials is the biggest challenge. Many possible new treatments for CF are in the pipeline, but they must be thoroughly tested to prove they work and are safe. This can only happen if enough people with CF participate in clinical trials.

To help recruit more people, the CF Foundation is making a special effort to give all people with CF the chance to be in clinical trials. We have increased the number of care centers who can offer clinical trials and we are striving to share the importance of participating in clinical trials with everyone who has CF.