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Clinical Trials: What You Need to Know

Knowing all you can about clinical trials can help you feel more certain when deciding whether to take part in one. The information in this section addresses many questions and concerns about clinical trials. It can help prepare you to discuss the pros and cons with your doctor and your family and decide whether being in a clinical trial is right for you.

- [Types and Phases of Clinical Trials](#)
 - [Deciding Whether to Be Part of a Clinical Trial](#)
 - [Protecting People in Clinical Trials](#)
 - [Being in a Clinical Trial](#)
 - [Finding a Clinical Trial](#)
-

Types and Phases of Clinical Trials

Clinical trials are studies to test new drugs, already approved drugs, devices, or other forms of treatments. Many clinical trials look at new ways to detect, diagnose, or measure the extent of disease. Some even look at ways to prevent diseases from happening. Researchers still use human volunteers to test these methods, and the same rules apply.

Doctors use clinical trials to learn whether a new drug, treatment, or combination works and is safe to use for people. Clinical trials are important in developing new treatments for serious diseases like cancer. All new treatments must go through clinical trials before being approved by the Food and Drug Administration (FDA). Cancer clinical trials can take years to complete. It can take months, if not years, to see if a cancer treatment does what it is meant to do.

Why do we need clinical trials?

Clinical trials show us what works (and what doesn't) in medicine and health care. They are the best way to learn what works in treating diseases like cancer. Clinical trials are designed to answer some important questions:

- Does the new treatment work in people? If it does, doctors will also look at how well it works. Is it better than treatment now being used? If it's not better, is it as good and cause fewer side effects? Or does it work in some people who aren't helped by current treatments?
- Is the new treatment safe? No treatment or procedure – even one already in common use – is without risk. But do the benefits of the new treatment outweigh the risks?
- Is this treatment better than the standard treatment given for this disease? Clinical trials help show if a new drug or treatment, or a new treatment combination, works better than what is now used.

Answering these questions, while giving as few people as possible an unknown treatment, often requires several clinical trials in different "phases." Each phase is designed to answer certain questions while keeping the people taking part as safe as possible. Results from these phases show if the new drug or treatment is reasonably safe and effective.

Pre-clinical (or laboratory) studies

Clinical trials are done only after pre-clinical findings suggest that the new drug or treatment is likely to be safe and will work in people.

Pre-clinical studies, also called laboratory studies, include:

- **Cell studies:** These are often the first tests done on a new treatment. To see if it might work, researchers look for effects of the new treatment on cancer cells

that are grown in a lab dish or a test tube. These studies may be done on human cancer cells or animal cancer cells.

- **Animal studies:** Treatments that look promising in cell studies are tested next on cancers in live animals. This gives researchers an idea of how safe the new treatment is in a living creature.

Pre-clinical studies give a lot of useful information, but not all that is needed. Humans and mice can be very different in the way they absorb, process, and get rid of drugs or treatments. A treatment that works against cancer in a mouse might or might not work in people. There could also be side effects and other problems that didn't show up when the treatment was used in mice but could show up in people.

If the pre-clinical studies are completed and the treatment still seems promising, the US Food and Drug Administration (FDA) must give permission before the treatment can be tested people.

The investigational new drug (IND) application

Before a clinical trial can be started, the research must be approved. An investigational new drug or IND application or request must be filed with the FDA when researchers want to study a drug in humans. The IND application must contain certain information, such as:

- Results from studies so that the FDA can decide whether the treatment is safe for testing in people.
- How the drug is made, who makes it, what's in it, how stable it is, and more.
- Detailed outlines for the planned clinical studies, called study protocols, are reviewed to see if people might be exposed to needless risks.
- Details about the clinical trial team to see if they have the knowledge and skill to run clinical trials.

The research sponsor must commit to getting informed consent from everyone on the clinical trial. They must also commit to having the study reviewed by an institutional review board (IRB) and following all the rules required for studying investigational new drugs

Phases of clinical trials

Clinical trials are usually conducted in phases that build on one another. Each phase is designed to answer certain questions. Knowing the phase of the clinical trial is important because it can give you some idea about how much is known about the treatment being studied. There are benefits and risks to taking part in each phase of a clinical trial.

Although there are clinical trials for devices as well as other diseases and treatments, drugs for cancer patients are used in the examples of clinical trial phases described here.

Phase 0 clinical trials: Exploring if and how a new drug may work

Even though phase 0 studies are done in humans, this type of study isn't like the other phases of clinical trials. The purpose of this phase is to help speed up and streamline the drug approval process. Phase 0 studies may help researchers find out if the drugs do what they're expected to do. This may help save time and money that would have been spent on later phase trials.

Phase 0 studies use only a few small doses of a new drug in a few people. They might test whether the drug reaches the tumor, how the drug acts in the human body, and how cancer cells in the human body respond to the drug. People in these studies might need extra [tests](#)¹ such as biopsies, scans, and blood samples as part of the process.

Unlike other phases of clinical trials, there's almost no chance the people in phase 0 trials will benefit. The benefit will be for other people in the future. And because drug doses are low, there's also less risk to those in the trial.

Phase 0 studies aren't widely used, and there are some drugs for which they wouldn't be helpful. Phase 0 studies are very small, often with fewer than 15 people, and the drug is given only for a short time. They're not a required part of testing a new drug.

Phase I clinical trials: Is the treatment safe?

Phase I studies of a new drug are usually the first that involve people. Phase I studies are done to find the highest dose of the new treatment that can be given safely without causing severe side effects. Although the treatment has been tested in lab and animal studies, the side effects in people can't be known for sure. These studies also help to decide on the best way to give the new treatment.

Key points of phase I clinical trials

- The first few people in the study get a very low dose of the treatment and are watched very closely. If there are only minor side effects, the next few participants get a higher dose. This process continues until doctors find a dose that's most likely to work while having an acceptable level of side effects.
- Phase I trials are also looking at what the drug does to the body and what the body does with the drug.
- Safety is the main concern. The research team keeps a close eye on the people and watches for any severe side effects. Because of the small numbers of people in phase I studies, rare side effects may not be seen until later phases of trials when more people receive the treatment.
- While some people may benefit from being on one, disease response is not the main purpose of a phase I trial,
- Placebos (inactive treatments) are not used in phase I trials.
- Phase I trials usually include a small number of people (up to a few dozen).
- Phase I trials most often include people with different types of cancer.
- These studies are usually done in major cancer centers.

Phase I trials carry the most potential risk. But phase I studies do help some patients. For those with life-threatening illnesses, weighing the potential risks and benefits carefully is key. Sometimes people choose to join phase I trials when all other treatment options have already been tried.

Phase II clinical trials: Does the treatment work?

If a new treatment is found to be safe in phase I clinical trials, a phase II clinical trial is done to see if it works in certain types of cancer. The benefit the doctors look for depends on the goal of the treatment. It may mean the cancer shrinks or disappears. Or it might mean there's a long period of time where the cancer doesn't get any bigger, or there's a longer time before the cancer comes back. In some studies, the benefit may be an improved quality of life. Many clinical trials look to see if people getting the new treatment live longer than most people do without the treatment.

Key points of phase II clinical trials

- A group of 25 to 100 patients with the same type of cancer get the new treatment in a phase II study. They're treated using the dose and method found to be the safest and most effective in phase I studies.
- Usually in a phase II clinical trials, everyone gets the same dose. But some phase II

studies randomly assign people to different treatment groups. These groups may get different doses or get the treatment in different ways to see which provides the best balance of safety and response.

- Placebos (inactive treatments) are not used in phase II trials.
- Phase II studies may be done at major cancer centers, community hospitals or even doctors' offices.

Larger numbers of patients get the treatment in phase II trials, so less common side effects may be seen. If enough patients benefit from the treatment, and the side effects aren't too bad, phase III clinical trials are begun.

Phase III clinical trials: Is it better than what's already available?

Treatments that have been shown to work in phase II clinical trials must succeed in one more phase before they're approved for general use. Phase III clinical trials compare the safety and effectiveness of the new treatment against the current standard treatment.

Because doctors do not yet know which treatment is better, study participants are often picked at random (called **randomized**) to get either the standard treatment or the new treatment. When possible, neither the doctor nor the patient knows which of the treatments the patient is getting. This type of study is called a **double-blind study**. Randomization and blinding are discussed in more detail later.

Key points of phase III clinical trials

- Most phase III clinical trials include a large number of patients, at least several hundred.
- These studies are often done in many places across the country (or even around the world) at the same time.
- Phase III clinical trials are more likely to be offered in local community hospitals and doctor's offices.
- These studies tend to last longer than phase I and II studies.
- Placebos may be used in some phase III studies, but they're never used alone if there's a treatment available that works. Sometimes, a patient who is randomly assigned to the placebo for part of the study will at some point be offered the standard treatment as well.

As with other trials, patients in phase III clinical trials are watched closely for side effects, and treatment is stopped if they're too hard to manage.

Submission for FDA approval: New drug application (NDA)

In the United States, when phase III clinical trials (or sometimes phase II trials) show a new drug is more effective or safer than the current treatment, a new drug application (NDA) is submitted to the Food and Drug Administration (FDA) for approval. The FDA reviews the results from the clinical trials and other relevant information.

Based on the review, the FDA decides whether to approve the treatment for use in patients with the illness the drug was tested on. If approved, the new treatment often becomes a standard of care, and newer drugs may be tested against it before they can be approved.

If the FDA feels that more evidence is needed to show that the new treatment's benefits outweigh its risks, it may ask for more information or even require that more studies be done.

Phase IV clinical trials: What else do we need to know?

Drugs approved by the FDA are often watched over a long period of time in phase IV studies. Even after testing a new medicine on thousands of people, all the effects of the treatment may not be known. Some questions may still need to be answered. For example, a drug may get FDA approval because it was shown to reduce the risk of cancer coming back after treatment. But does this mean that those who get it are more likely to live longer? Are there rare side effects that haven't been seen yet, or side effects that only show up after a person has taken the drug for a long time? These types of questions may take many more years to answer, and are often addressed in phase IV clinical trials.

Key points of phase IV clinical trials

- Phase IV studies look at drugs that have already been approved by the FDA. The drugs are available for doctors to prescribe for patients, but phase IV studies might still be needed to answer important questions.
- These studies may involve thousands of people.
- This is often the safest type of clinical trial because the treatment has already been studied a lot and has likely been given to many people. Phase IV studies look at safety over time.

- These studies may also look at other aspects of the treatment, such as quality of life or cost effectiveness.

You can get the drugs used in a phase IV trial without being in a study. And the care you would get in a phase IV study is very much like the care you could expect if you were to get the treatment outside of a trial. But in phase IV studies you're helping researchers learn more about the treatment and doing a service to future patients.

Hyperlinks

1. www.cancer.org/treatment/understanding-your-diagnosis/tests.html

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Deciding Whether to Be Part of a Clinical Trial

Each clinical trial has benefits and risks. Before you decide to be in one, you need to have all your questions answered. Some people take notes, record meetings with the clinical team, or bring a friend with them to help remember the answers and think of other questions.

Questions to ask before joining a clinical trial

- What [phase](#) is this clinical trial in?
- Why is this study being done?
- How long do I have to make this decision?
- What's likely to happen if I decide to take part or not take part in the clinical trial?
- Will the researchers work with my cancer doctor? Who will be in charge of my care?
- Who will I get in touch with if I have problems, questions, or concerns?
- What are my other options (standard treatments, other clinical trials)? What are the pros and cons of each?
- How much do you know about this treatment? About clinical trials in general?
- What were the results in past studies of this treatment? How likely are they to apply to me?
- Is there anything else I can read about this clinical trial?
- What kinds of treatments and tests would I need to have? How often are they done?
- Would I need to plan on extra time or travel?
- What side effects might I expect from the trial treatment? Are there other risks? How do these side effects compare to the side effects from standard care for my illness?
- How will we know if the treatment is working?
- Will I have to be in the hospital for any part of the trial? If so, how often, for how

- long, and who will pay for it?
- Will I still be seeing my regular cancer doctor?
 - Will I have to pay for anything? Will my insurance cover the treatment?
 - If I am harmed as a result of the research, what treatment will I be entitled to?
 - How long will I be in the clinical trial? How long will the clinical trial last?
 - Will I still be able to work if I am in the clinical trial?
 - Are there reasons I could be removed from the clinical trial? Are there reasons the clinical trial might be stopped early?
 - Is long-term follow-up care part of the trial? What would it involve?
 - If the treatment is working for me, can I keep getting it even after the clinical trial ends?
 - Can I talk to other people taking part in the clinical trial?
 - Will I be able to find out about the results of the clinical trial?
 - Is there anything else I can read about this clinical trial?

You might find it helpful to include trusted friends and family members in your decision-making process. They might ask questions you hadn't thought of and can help you make sure that you're choosing what's right for you. Also, getting a [second opinion](#)¹ from a doctor who's not part of the study may help you decide if a certain study is the best option for you.

Risks versus benefits

Each clinical trial has its own benefits and risks. But for the most part, clinical trials (other than phase 0) have some of the same potential benefits:

- You might help others who have the same disease by helping to advance cancer research.
- You could get a treatment that's not available outside of the trial. This treatment might be safer or work better than current options.
- This may increase the number of your treatment options.
- You may feel more control by taking a more active role in your health care.
- You'll likely see your cancer care team more often so that they can monitor your disease and check for side effects of the new treatment.
- Some study sponsors may pay for part or all your medical care and other expenses during the trial. (This isn't true for all clinical trials.)

Some possible risks of being in a clinical trial can include:

- The new treatment may have unknown side effects or other risks which might be worse than those from standard treatments.
- The new treatment may not work for you even if it helps others.
- You may need to have more doctor visits or testing which may require more time and travel.
- If you take part in a [randomized clinical trial](#), you may not have a choice about which treatment you get. If the study is blinded, you (and maybe your doctor) won't know which treatment you're getting. (This information will be available to the clinical trial team as needed for your safety).
- Insurers may not cover all costs of the clinical trial. They usually do cover the costs of what would normally be standard care. Be sure to talk to your insurance provider and someone involved with the clinical trial before you decide to take part.

Common concerns about clinical trials

Most people have some concerns about taking part in a clinical trial because they're not really sure what it will mean for them. Get as much information as you need to make the choice that's right for you.

Will there be risks?

Yes, all clinical trials have risks. But any medical test, treatment, or procedure has risks. The risk may be higher in a clinical trial because there are more unknowns. **This is especially true of phase I and II clinical trials**, where the treatment has been studied in fewer people.

Perhaps a bigger question is if the possible benefits outweigh the risks. People with cancer are often willing to accept a certain amount of risk for a chance to be helped. But it's always important to be clear on what this chance is. Ask your doctor to give you an idea of what the benefits may be and which is likely for you. Some people may decide that any chance of being helped is worth the risk, while others may not. Others may be willing to take certain risks to help others.

Will I be a “guinea pig?”

You will not be a guinea pig, but it's true that the purpose of a clinical trial is to answer a medical question. People who take part in clinical trials may need to do extra things or have certain tests done as part of the clinical trial.

But this doesn't mean that you won't get excellent care while in the study. In fact, most people enrolled in clinical trials welcome the extra attention they get from their cancer care team.

Studies have shown that people with cancer who felt well informed before they took part in a clinical trial had less regret after the study than those who felt unsure. That's why it's important to take your time, ask questions, and feel good about your decision.

Will I get a placebo?

A placebo is a fake pill or treatment used in some types of clinical trials to help make sure results are from the new treatment or drug. A placebo pill is sometimes called a "sugar pill." Placebos are rarely used alone in clinical trials unless there is no known effective treatment. Most cancer clinical trials do not use placebos unless they are given along with an active drug. It's unethical to give someone a placebo instead of a treatment that's known to work.

There are some types of cancer that no treatments have proven to help. In rare cases, testing a new treatment against a placebo might be needed to prove that the treatment is better than nothing at all. The very least you should expect from any clinical trial is to be offered the treatment standard of care.

Can my doctor or I pick which group I'm in?

Not for studies that are randomized. This means that each person in the study gets assigned by chance to either the treatment group or the control group (who get the best current treatment). Randomization helps decrease the chance that the people in one group will be so different from the other that it could affect outcomes. Randomization helps to make sure that the groups have people in similar states of health, so the results are not affected by differences between the groups. If people could choose which treatment they got, the study results might not be as accurate.

Some people find the concept of randomized clinical trials distressing, since neither the patient nor the doctor can choose which group the patient is in. This can be especially true if a trial is looking at totally different treatments and a person believes that one is better than the other. But remember, doctors are doing the study because they really don't know which one is better. And sometimes taking part in such a study is the only way a person has a chance of getting a new treatment.

Will I know which group I'm in? Will my doctor know?

Each study is different. In a **blinded** study, the patient doesn't know which treatment they're getting. In a **double-blinded study**, neither the patient nor the doctor knows which treatment is being used. Not knowing what you're getting can be hard. Your doctor can always find out which group you're in if there's an important medical reason (such as a possible drug reaction), but it may result in your being removed from the study. Blinding reduces the risk that the doctor or patient's beliefs about the new treatment will affect their evaluation of response or side effects.

Will my information be kept confidential?

As much as possible, your personal and medical information will be kept confidential. Of course your cancer care team needs this data to give you the best possible care, just as they would if you were not in a clinical trial.

Information that's needed for the clinical trial, such as test results, is put on special forms and into computer systems. This is only shared with the people who analyze the study results. Your data is given a number or code – your name isn't on the forms or in the study system. Sometimes, members from the research team or from the FDA might need to look at your records to be sure the data they were given is correct. But your personal information isn't given to them and is never used in any published clinical trial results.

What about cost? Will my insurance cover it?

In most cases, the [study sponsor](#) provides the new treatment at no cost and pays for any special tests or extra doctor visits. Some sponsors may pay for more. For example, some might pay you back for travel time and mileage. It's important to find out what will be paid for before you decide to get involved in any clinical trial.

The [Affordable Care Act](#)² requires that health insurance covers the routine costs of care for people who are in approved clinical trials. "Routine costs" are those that anyone who is getting treated for your kind of cancer would have covered. Insurers are not allowed to drop or limit coverage because a person chooses to take part in a clinical trial. This applies to all clinical trials unless the insurance plan is "grandfathered." (Grandfathered plans are those that a person was enrolled in on or before March 23, 2010 and which has not decreased benefits or increased costs.)

The law requires insurance coverage for phase I, II, III, or IV clinical trials related to prevention, detection, or treatment of cancer or other life-threatening disease if the clinical trial meets one of these requirements:

- It's federally funded (any US federal agency such as the National Cancer Institute, Centers for Disease Control, Department of Defense, etc.)
- It's covered under an investigational new drug application (IND) that's reviewed by the FDA
- It's does not need an IND application.

Insurers do not need to pay for:

- The treatment, device, or service that's being studied. This is usually paid for by the trial's sponsor
- Items and services only needed for data collection and analysis and not used in direct patient care
- Any service that's clearly not in line with standards of care for a certain type of cancer. This is why it is important to know what the clinical trial may not pay for so you can check if your insurance will.

Medicare coverage for clinical trials

If you have Medicare, it pays for many of the routine medical costs for people with cancer who are in approved clinical trials. This is true no matter where in the United States you live. Medicare normally covers any cancer care when it's part of either:

- A clinical trial for the diagnosis and treatment of cancer; or
- A clinical trial funded by the National Cancer Institute (NCI), NCI-Designated Cancer Centers, NCI-Sponsored Clinical Trials Cooperative Groups, or another federal agency that funds cancer research

Cancer prevention trials are not currently covered by Medicare. If you're not sure if your trial meets all the requirements, discuss these concerns with your doctor or call Medicare (1-800-633-4227).

What you can do to find out more about costs

Gather as much information as you can about the clinical trial and contact your insurance provider to find out about payment. Many insurers may not be able to give you a simple yes or no answer, because they may review claims on a case-by-case basis. They'll also want to be sure that the doctors supplying the main part of your cancer care are "in network."

If your insurance will not pay for parts of the clinical trial, ask your doctor or the research coordinator about other options. Sponsors may be willing to cover some of the costs your insurance does not.

Should I agree to take part in a clinical trial?

This can be a very tough question. The answer won't be the same for everyone. When trying to decide, first ask yourself some questions.

- Why do I want to take part in a clinical trial?
- What are my goals and what do I expect if I decide to take part? How realistic are these?
- How sure are my doctors about my future if I decide to take part (or not take part) in this clinical trial?
- Do I have all the information I need to make an informed decision?
- Have I weighed the benefits against the risks?
- Have I thought about other factors, such as travel, time, and money?
- Have I looked at my other options?

Some of these questions may not have clear answers but should help you start thinking about these issues. Each person's situation is unique, and each person's reasons for wanting or not wanting to take part in a trial may be different.

Hyperlinks

1. www.cancer.org/treatment/finding-and-paying-for-treatment/choosing-your-treatment-team/seeking-a-second-opinion.html
2. www.cancer.org/treatment/finding-and-paying-for-treatment/understanding-health-insurance/health-insurance-laws/the-health-care-law.html

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Protecting People in Clinical Trials

Several safeguards are used to help protect people who take part in clinical trials. There are still risks involved with any study, but these safeguards try to reduce the risk as much as possible.

Research involving people is based on three values described in the Belmont Report:

- **Respect:** All people should be respected. They have the right to choose what treatments they get.
- **Beneficence:** Protect people from harm by giving the most benefit with limited risks
- **Justice:** Try to make sure that all people share the benefits and burdens equally

Research staff and government groups are charged with making sure that these values are followed in clinical trials. A key part of patient protection is the informed consent process, which is described in [Being in a Clinical Trial](#).

Safeguards in institutions

All clinical trials must be reviewed by special groups to make sure that people in the trials are protected.

Institutional review boards (IRBs)

IRBs make sure that people in clinical trials are protected and that federal laws are followed. In turn, the federal Office of Human Research Protections (OHRP; see below) makes sure that IRBs follow laws that direct their process. Before starting, researchers must send the clinical trial protocol (the plan that describes the study in detail) to the IRB for review. The IRB must decide if the study looks at a worthwhile question and ensures the safety of the people on the trial.

The IRB also makes sure that the [informed consent](#)¹ form that people sign before going on a trial is accurate, complete, and easy to understand. Once a clinical trial begins, the IRB watches over it to look for problems. If you are in a clinical trial, you can contact the IRB with any questions or concerns.

Data safety monitoring boards (DSMBs)

Data safety monitoring boards (DSMBs) are used for [phase III](#) (and some earlier phase) clinical trials. The job of the DSMB is to watch the progress and results of the clinical trial. The DSMB can stop a clinical trial before it is done if:

- It becomes clear that the new treatment is much more (or much less) effective to allow all people on the clinical trial to get the better treatment
- Safety concerns come up, such as risks being much greater than benefits, so that no one else is exposed to possible harm.

The clinical investigator

The clinical investigator is in charge of all parts of a clinical trial. In some settings this person is called the principal investigator, or PI. The main responsibility for patient safety in a clinical trial belongs to the clinical investigator. This includes letting the study sponsor know right away when severe side effects occur.

Government agencies

Office of Human Research Protections (OHRP)

The OHRP is the government's main protector of people's safety in clinical trials. The OHRP makes sure that the rules of informed consent, IRBs, and participation of people with special needs are followed. OHRP can stop clinical trials when problems are found. OHRP also teaches research sites and individuals to help them follow current clinical trial standards.

Food and Drug Administration (FDA)

The FDA has the final say about whether a new treatment is approved to be given to patients. Once phase III clinical trials are completed, the FDA reviews the results of all trials and decides if the new treatment is safe and effective enough to be approved.

The FDA is also involved during the clinical trial. Clinical trials for any new drug, device or treatment must be approved by the FDA before they can begin. The FDA also inspects (audits) sites doing clinical trials, especially if there's reason to think that the site is not following proper procedures. If serious problems are found, the FDA can prevent a site or doctor from doing any further research.

It is important to note that the FDA is not involved in the study of all treatments. Clinical trials of treatments that are already on the market do not have to follow the same FDA rules. And substances considered to be [dietary supplements](#)² do not need FDA approval. (Dietary supplement makers don't have to prove their products are safe and effective.)

National Cancer Institute (NCI)

The NCI, part of the National Institutes of Health (NIH), pays for many cancer clinical trials. Plans for trials must be approved by the NCI before they get funded. The NCI also audits each site doing NCI-sponsored research to make sure the right procedures are being followed.

Hyperlinks

1. www.cancer.org/treatment/finding-and-paying-for-treatment/understanding-financial-and-legal-matters/informed-consent.html
2. www.cancer.org/treatment/treatments-and-side-effects/complementary-and-alternative-medicine/dietary-supplements.html

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Being in a Clinical Trial

People usually don't think about being in a clinical trial until they have a serious illness. Medical advances often make the news but you usually don't hear about the thousands of people who are helped each year because they took part in a clinical trial. You also aren't likely to hear about the millions of others who benefit because of the people who were in clinical trials.

Sometimes clinical trials are slowed or do not get completed because not enough people are enrolled. This is especially true for clinical trials that need to enroll racial minorities and those with other diseases (such as HIV infection and kidney disease). Clinical trial groups are taking steps to recruit a more diverse group of people.

Eligibility, or inclusion and exclusion criteria

Before you can join a clinical trial, you must meet the requirements (eligibility criteria). All clinical trials have criteria about who can and cannot be in them.

- The factors that **allow** a person to sign up for a study are called **inclusion** criteria.
- The factors that can **prevent** a person from joining a study are called **exclusion** criteria.

These criteria are used to make sure that only people who can safely take part in the clinical trial are included.

For cancer clinical trials, the inclusion and exclusion criteria usually have to do with:

- The type of cancer a person has
- The stage (extent) of the cancer
- Previous treatments a person had
- The length of time since a person last had treatment
- Results of certain lab tests
- The medicines a person is taking
- Other medical conditions the person has
- Any previous history of another cancer
- A person's activity level (often called the performance status)

Other factors, such as a person's age or sex, may also be part of the criteria. There are usually other criteria for each study as well.

Informed consent or informed permission

The first thing you will be asked to do is provide written, informed consent. During the informed consent process, the researchers (doctors or nurses) will explain the details of the trial to you and answer your questions and concerns. If you're looking at a study for your child, this process is much the same but may be called informed permission.

You will then be given a written consent form. Consent forms for clinical trials usually include the following:

- The reason for the study (what the researchers hope to find out)
- Who is eligible to take part
- What's known about the new treatment
- The possible risks and benefits of the new treatment
- Other treatments that may be an option for you
- The design of the study (whether it's randomized, double blinded, etc.)

- How many and what types of tests and doctor's visits will be needed
- Who must pay for the costs of the clinical trial (tests, doctor's visits, etc.) and for costs if you need extra care as a result of the clinical trial
- A statement about how your identity will be protected
- A statement about the study being voluntary and your right to leave the study at any time without fear of losing care that you would receive outside of the trial
- Contact information in case you have questions before, during or after the study

Before you sign the consent form, ask questions. Be sure someone from the research team goes over the form with you. Consent forms are not all easy to understand, and there may be words or ideas that are confusing. You may want to bring someone along with you to help make sure all your concerns are addressed.

Parents must sign the form for their child, and in most cases older children must also agree before they take part. (This is called **assent**.) In general, children's rights are very like those of adults, including the right to leave the study any time they want.

Be sure you understand what's involved and what's expected of you. Review what you understand about the study with your doctor or nurse to make sure you've got it right.

Finally, don't feel rushed to make a decision. If you are eligible for a clinical trial, you always have the [right to choose](#) if you will take part. The level of care you get should not be affected by your decision.

Take the consent form home with you if you want to. Ask trusted family members and friends what they think. Some people may want to get a [second opinion](#)¹ about the study from another doctor, too.

Taking part in the study

Once you've signed the consent form, you'll probably need to have [blood tests](#)² and/or [imaging tests](#)³ before you start treatment. Usually, you will be asked about your medical history and examined physically. These are needed to be sure that you meet the eligibility criteria and help ensure your safety.

What happens next may seem like getting a normal treatment, but you may need more tests and visits to the doctor. These tests are to find out how the treatment is going and how you're doing. The doctors and nurses may examine you more often and will want to know if you're having any side effects (called **adverse events**) while being treated.

Because the possible problems may not fully be known, it's very important to let the research team know if you have any symptoms that are not normal for you. The research team will decide if symptoms you're having are related to the clinical trial, and if they need to be treated or your treatment needs to be changed.

What if I want to leave the study early?

You will be told many times before you agree to be in a clinical trial that taking part is always voluntary. This is an important point. You (or your child) have the right to stop taking part in the study at any time, for any reason. You'll want to know how quitting the study might affect your health and what other treatment options you have. You should also tell the clinical trials team that you're quitting and why. The clinical trials team may ask to continue to follow up with you for a certain length of time to look for any long-term effects of treatment. This helps find unexpected problems and can make sure you are safe, even though you're no longer part of the clinical trial. Your doctors will still take care of you to the best of their ability.

People stop being part of clinical trials for many reasons. You might quit because it's not working for you. You might leave the trial because of severe side effects. Or you just decide you no longer want to be in it. **THIS IS YOUR CHOICE** and not a problem.

Also, the clinical trial itself may be ended early if the treatment being studied is proven to work or NOT work as well as the standard treatment. Sometimes, the new treatment is found to have side effects that cannot be managed.

What if I'm not eligible for a clinical trial?

Some people are too sick or have other problems that keep them from taking part in clinical trials.

Other people may be interested in a certain treatment that's only available in clinical trials, but they don't meet the eligibility criteria. In some of these cases, a person's doctor may ask the study sponsor if they can get an eligibility waiver or special exception to allow the person into the study, even though they don't meet all of the criteria. This decision is usually made by the trial's clinical investigator, who may consult with others involved in the study about the request. If the clinical investigator agrees to allow a waiver, the person is treated according to the study protocol (the same tests, doctor's visits, follow-up, etc.), but that person's results are not included in the final study results.

In some cases, studies may have already enrolled enough people and aren't taking any

more participants.

Sometimes, there are ways to get access to treatments that are in late-phase clinical trials but not yet approved by the FDA. These are usually referred to as **expanded access** or compassionate use programs. In recent years the FDA has broadened these programs to allow some patients who urgently need these treatments to be able to get them. For more, see [Compassionate Drug Use](#)⁴.

Hyperlinks

1. www.cancer.org/treatment/finding-and-paying-for-treatment/choosing-your-treatment-team.html
2. www.cancer.org/treatment/understanding-your-diagnosis/tests/understanding-your-lab-test-results.html
3. www.cancer.org/treatment/understanding-your-diagnosis/tests.html
4. www.cancer.org/treatment/treatments-and-side-effects/clinical-trials/compassionate-drug-use.html

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Finding a Clinical Trial

At one time, clinical trials were done only at major medical centers. Many patients had to travel a long way and were treated by doctors they didn't know very well. For some trials, especially with phase I and some phase II studies, this can still be the case. But this isn't always a bad thing. Some people prefer to be treated in major cancer centers because of their experience, reputation, and resources. The hassles of traveling must be weighed against the chance of being helped by the treatment.

Today, patients have more options. This may include staying closer to home during a study or even staying with their own doctors. Your doctor may or may not be involved in clinical trials. If they are, they might have one that is a good fit for you. Whether they have the right study for you is a question worth asking. And know that while clinical trials are now done in many places, the same rules are there to protect patients.

Most people who take part in clinical trials hear about them from their doctors. But you don't have to wait for your doctor to bring up a clinical trial. People with cancer can also look for clinical trials online or in other places to find more options for treatment.

Where to get information about current clinical trials

There are groups that provide ways to search for clinical trials on their websites. Many

of these groups also have people who can help you with your search.

Search results often include a description of each study, factors that people must meet to go into the trial ([eligibility](#)), and a contact person. These sites also help you focus your search using factors like your type and stage of cancer, the [kind of treatment](#)¹ you're looking for (chemotherapy, immunotherapy, radiation therapy, etc.) and where you live.

General cancer clinical trial listings

There are several websites that give details about clinical trials for all types of cancer. The clinical trials on each of these sites may be a bit different, but most trials will be on all sites. Many of these groups also have a phone number you can call for help with your search. Here are some of the most often used resources.

- National Cancer Institute (NCI) provides an online search tool for cancer clinical trials at cancer.gov/about-cancer/treatment/clinical-trials². You can ask for help with a search by calling 1-800-4-CANCER (1-800-422-6237).
- Center for Information and Study on Clinical Research Participation (CISCRP) provides an online search tool at ciscrp.org³. You can also get help with searches at 1-877-MED HERO (1-877-633-4376).
- National Institutes of Health (NIH) has a large database of clinical trials at clinicaltrials.gov⁴. Not all the studies listed are cancer clinical trials.
- [EmergingMed Clinical Trial Navigator Service](#)⁵ includes an online search tool to look for clinical trials. You can also get help from a clinical trial navigator through their website.

Clinical trial listings, by cancer type

Many cancer advocacy groups offer help in finding clinical trials that might be a good fit for you. Because many of these groups focus on a specific type of cancer, they can help you find trials for your type of cancer. A list of some of these services can be found on the cancer.net⁶ website. If you don't see your type of cancer on this list you can search the internet for advocacy groups.

Private companies

Clinical trials are also sponsored by pharmaceutical and biotechnology companies. These companies must prove their medicines or devices are safe and effective before

they can be marketed. They may list the studies they're sponsoring on their websites or offer toll-free numbers so you can call and ask about clinical types.

Finally, there are doctors, medical centers, foundations, volunteer groups, and other non-profit groups that sponsor clinical trials.

Some also offer matching systems for the studies they sponsor. This can be helpful if you're interested in a specific experimental treatment and know which company is developing it.

Note: This is not a complete list of all clinical trial sites. You can find others listed on the internet. Before choosing a site to use, you should check to see if there is a fee, how they keep your personal information safe, how they choose the clinical trials to list, and if they receive money to rank or list trials.

The clinical trial study protocol

The study protocol is the written plan for a clinical trial. It's sent to the Food and Drug Administration (FDA) and to an institutional review board (IRB) for [approval](#) before a new treatment can be studied in people.

A protocol describes:

- Why the clinical trial is being done
- Information about the treatment being tested (such as names and doses of drugs to be used) and results of any clinical trials done before
- The [phase of the clinical trial](#) and how many people will be in it
- Who may participate
- How the treatment is given
- What tests will be done and when they'll be done
- Other details that will be collected on participants
- How long the trial will last

The lists of clinical trials that are available online often include summaries of these protocols with key points. Research team members may also have protocol summaries or other information they can share with you.

Study protocols can be very long and complex. They aren't written with patients in mind, so making sense of them can be tough. Often, the most important information for patients is the [eligibility criteria](#) and any details known on the new treatment.

Clinical trial lists may not contain all of the eligibility criteria. If you've found a study you think you might qualify for, you should be able to find contact information for someone who can give you a full list of the requirements.

I think I'm eligible. Now what?

Once you've found a clinical trial and think you are eligible for it, deciding if it's the right one for you can still be hard. There may even be more than one trial that looks like an option. It's important to learn as much as you can.

Talk with someone linked to the clinical trial. This could be the clinical or principal investigator (PI) – the person in charge of the study – or a research coordinator. Research coordinators are usually nurses. One of their jobs is to make sure that people understand the trial and meet eligibility criteria before they become part of a study. They also make sure that the study protocol is followed for each patient. The research coordinators often serve as links between study patients and their doctors.

Both PIs and research coordinators should be able to answer your questions about the clinical trial. They can give you answers about the clinical trial, but they probably won't have information about other studies you might be thinking about. What's more, they could be biased (even if they don't mean to be) toward their own study.

Talk to your cancer or primary care doctor about the clinical trials you're looking at. Share the details you have so that your doctor can help you figure out what might be right for you. No doctor knows about every clinical trial being done, but your doctor knows your medical situation best and can probably tell you if the study is worth thinking about. This can take some time, so you might need to make a special appointment.

You might also want to get a [second opinion](#)⁷ from a doctor not connected to the clinical trials you're looking at. Doctors who are well known in their fields usually know about the latest experimental treatments, and they may be able to point to those that look like a better fit for you.

You can find some information on your own. Try to find out if the new treatment has been studied before or if it's now being studied in other diseases including whether any results are available.

Finally, talk to friends and family members. While the final decision is yours, their ideas may give you insight into things you hadn't thought about.

Hyperlinks

1. www.cancer.org/treatment/treatments-and-side-effects/treatment-types.html
2. www.cancer.gov/about-cancer/treatment/clinical-trials
3. www.cisrcp.org/
4. www.clinicaltrials.gov
5. app.emergingmed.com/emed/home
6. www.cancer.net/research-and-advocacy/clinical-trials/finding-clinical-trial
7. www.cancer.org/treatment/finding-and-paying-for-treatment/choosing-your-treatment-team/seeking-a-second-opinion.html

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