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Clinical Trials: What You Need to Know

Knowing all you can about clinical trials can help you feel better when deciding whether or not to take part in one. This guide addresses many basic questions and concerns about clinical trials so that you will be better prepared to discuss this option with your doctor and your family. It can help you decide which questions you need to ask and what the answers may mean for you.

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The Basics of Clinical Trials

Clinical trials are studies in which people volunteer to test new drugs or devices. Doctors use clinical trials to learn whether a new treatment works and is safe for people. These kinds of studies are needed to develop new treatments for serious diseases like cancer.

Clinical trials are vital in studying all aspects of medicine, not just cancer. The stakes may seem higher when researching medicines to treat cancer, but all new treatments (drugs and medical devices) must go through clinical trials before being approved by the FDA.

Who can be in clinical trials?

People of all ages can take part in clinical trials. If you [find a clinical trial](#) that interests you, there are [steps that must be taken](#) to join the study.

For a child to be enrolled in a clinical trial, the parents or guardians must decide if they want their child to take part. If the parents give permission, older children are usually asked whether they wish to take part. This process is called *assent*. In most cases, a child can refuse, even if the parents are willing to permit it. The process of considering a clinical trial is much the same for the parents of a child as it is for an adult.

There's always uncertainty when you're thinking about a clinical trial. Part of it is that the doctors in charge of a clinical trial don't know ahead of time how things will turn out. If they did, there would be no need for the study in the first place. So there's no simple answer to the question, "Should I take part in a clinical trial?"

Most people don't pay much attention to clinical trials until they have a serious illness. Medical breakthroughs (the results of clinical trials or other kinds of research) often make the news, but you usually don't hear about clinical trials themselves unless something has gone wrong in one of them. For instance, the media quickly reports it any time a volunteer in a study is harmed.

What you usually don't hear about in the news are the thousands of people who are helped each year because they decided to take part in a clinical trial. You also aren't likely to hear about the millions who benefit from others' participation in clinical trials.

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 best in treating diseases like cancer. Clinical trials are designed to answer 2 important questions:

- **Does the new treatment work in humans?** If it does, doctors are also looking at how well it works. Is it better than what's now being used to treat a certain disease? If it's not better, is it at least as good, while perhaps causing 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 entirely without risk. But do the benefits of the new treatment outweigh the possible risks?

Answering these questions, while exposing as few people as possible to an unknown treatment, often requires several different clinical trials. They're usually grouped into "phases." [Clinical trials in each phase](#) are designed to answer certain questions, while trying to make sure the people taking part are kept as safe as possible. Every new treatment is tested in 3 or more phases of clinical trials before being considered reasonably safe and effective.

New treatments have to pass many tests before they get to you.

Clinical trials are only a small part of the research that goes into developing a new treatment. Drugs of the future, for example, first have to be discovered or created, purified, described, and tested in labs (in cell and animal studies) before ever reaching human clinical trials. Of all the substances that are tested in these early stages, very few are promising enough to be tested in humans.

On average, a new cancer drug has been studied for at least 6 years before it even makes it to clinical trials. But the major holdup in making new cancer drugs available is how long it takes to complete clinical trials themselves. It takes an average of about 8 years from the time a cancer drug enters clinical trials until it's approved.

Why so long? To be sure it's safe and effective, researchers look at each new treatment in several different studies. Only certain people are eligible (meet the requirements) to take part in each clinical trial. And cancer clinical trials take years to complete. It takes months, if not years, to see if a cancer treatment works in any one person. And figuring out if a drug really improves survival can take a very long time.

The biggest barrier to completing clinical trials is that not enough people take part in them. Fewer than 5% of adults (less than 1 in 20) with cancer will take part in a clinical trial. But clinical trials are much more commonly used to treat children with cancer. In fact, 60% of children under age 15 participate in clinical trials. This is one reason that survival rates for childhood cancer have increased so dramatically in the last few decades.

The main reason people give for not taking part in a clinical trial is that they didn't know the studies were an option for them. But there are many other reasons. Some people want to take part but don't meet the requirements. Some are uncomfortable with the idea of being a volunteer in a study. Others worry that they won't be treated fairly or could be harmed by an unproven treatment.

How does a treatment qualify to be used in a clinical trial?

Before a clinical trial can be done it must be decided whether it's ethical to ask patients to volunteer for the experimental treatment. Has the study been designed, as much as possible, to make sure the people in it will be safe? Will the volunteers get a treatment that's at least as good as, and maybe even better than, what they would get if they didn't volunteer for the study?

Scientific panels are set up to review and approve all clinical trials to make sure questions like these are answered before the researchers are allowed to sign up patients.

Pre-clinical (or laboratory) studies

Clinical trials are done only after *pre-clinical* studies suggest that the proposed 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 they don't give all the answers that are needed. After all, humans and mice can be very different in the way they absorb, process, and get rid of substances. A treatment that works against cancer in a mouse may or may not work in people. And there could be side effects and other problems that didn't show up when the treatment was used in mice.

If the pre-clinical studies are completed and the treatment still seems promising, the US Food and Drug Administration (FDA) must give permission to test it in humans.

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,

as described below. The FDA reviews this information before human clinical trials start. Here's some of the information required on an IND request:

Pre-clinical studies: Results from studies, including those on animals, allow the FDA to decide whether the product is reasonably safe for testing in humans. This part may also include any experience with the drug in humans (if the drug has been used or studied in another country, for example).

Manufacturing information: This explains how the drug is made, who makes it, what's in it, how stable it is, and more about the physical qualities of the drug. The FDA uses this information to decide whether the company can make batches of the drug that will always be exactly the same.

Clinical protocols and investigator information: Detailed outlines for the planned clinical studies, called *study protocols*, are looked at to see if the study might expose subjects to unnecessary risks. Information on the clinical investigators who will supervise the study is reviewed to find out if they're qualified to run clinical trials. Finally, the research sponsor must commit to getting informed consent from the research subjects, having the study reviewed by an institutional review board (IRB), and following all the rules required for studying investigational new drugs

Some facts about clinical trials to keep in mind

Taking part in any clinical trial is voluntary.

You always have the right to choose whether you will take part in a clinical trial for which you meet the criteria. The level of care you get should not be affected by your decision.

You also have the right to leave a clinical trial at any time, for any reason. If you decide to leave the study, talk to your doctor first. 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 research group that you're quitting and why. The clinical trials team may ask that you agree to continue to be watched for a certain length of time to look for any long-term effects of treatment.

Not all clinical trials study treatments.

Many clinical trials look at new ways to detect, diagnose, or learn the extent of disease. Some even look at ways to prevent diseases from happening in the first place. Researchers still use human volunteers to test these methods, and the same general rules apply.

Clinical trials are not all drug trials.

Many clinical trials test other forms of treatment, such as new surgery or radiation therapy techniques, or even complementary or alternative medicines or techniques.

Clinical trials are used to study approved drugs, too.

Even after a drug has been approved for use against a type of cancer, doctors sometimes find it works better when given a certain way or when combined with other treatments. It may even work on a different type of cancer. Clinical trials are needed to study these possibilities, too.

Clinical trials and placebos

A placebo is a sham pill, inactive ingredient, or fake treatment used in some types of clinical trials to help make sure results are unbiased. A placebo pill is sometimes called a “sugar pill.”

Over the years, doctors have found that some people begin to feel better even if they just think they’re being treated. Although this effect tends to be brief, and doesn’t really affect cancer, it can make a new treatment seem to help. Sometimes, people who know they’re getting placebo don’t report all the health problems that come up, while those on the treatment do. This can make the treatment look like it has worse side effects than it does. A placebo control group keeps people from knowing if they’re getting the treatment being studied, and makes the results more likely to be valid.

Placebos are rarely used alone in cancer research unless no known effective treatments exist. It’s not ethical to have someone take a placebo if there’s a treatment available that works. When cancer clinical trials compare treatments, they compare the new treatment against the current standard (proven) treatment. At times, a study may be designed so that patients don’t know which one they’re getting, but they know they’re getting treatment that at the very least meets the current standard of care.

In some clinical trials, the doctors want to learn if adding a new drug to the standard therapy makes it work better. In these studies, some patients get the standard drug(s) and the new one being tested, while other patients get the standard drug(s) and a placebo. But none of the patients would get only a placebo. Everyone gets standard treatment if there’s a standard treatment available. For more information about placebos and how they might be used in some studies, see [Placebo Effect](#)¹.

Who sponsors and runs clinical trials?

The National Cancer Institute (NCI) sponsors (pays for) a good portion of the thousands of cancer clinical trials going on in the US at any given time. The NCI is a part of the National Institutes of Health (NIH), which is funded by US tax dollars. These studies are often run through the NCI's National Clinical Trials Network (NCTN), which are networks of doctors and institutions across the country that specialize in a certain aspect of cancer.

NCI Cancer Centers also conduct research at their facilities across the United States. Government agencies other than NCI, including parts of the Department of Veterans Affairs and the Department of Defense, often sponsor cancer clinical trials. And, there are doctors, academic medical centers, foundations, volunteer groups, and other non-profit organizations that sponsor clinical trials, too.

The other main sponsors of clinical trials are pharmaceutical and biotechnology companies, which must prove their medicines or devices are safe and effective before they can be marketed.

Researchers conduct clinical trials in many different settings. Major cancer centers are often the focal points of clinical trials research. Because they usually have the most advanced facilities and highly trained staffs, they can conduct all phases of clinical trials.

Community hospitals across the country also take part in clinical trials, although these are usually phase II or III studies. Many of these hospitals are part of the [NCI's Community Oncology Research Program \(NCORP\)](#)², which means they work with an NCI cancer center or the National Clinical Trials Network. NCORP members conduct the same clinical trials across the country. Community hospitals may conduct privately sponsored and other types of studies, too.

Doctors in private practice can also be involved in clinical trials, either as members of cooperative groups or by being actively involved in privately sponsored research.

What this may mean for you

At one time, clinical trials were done only at major medical centers. This often meant that patients had to travel long distances and were treated by doctors they didn't know very well. This is sometimes still the case, especially with phase I and some phase II studies. Of course, this isn't always a bad thing. Many people prefer to be treated in major cancer centers because of their experience, reputation, and resources. Ultimately, 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 he or she is, you may be eligible for one of them. Whether this is the right study for you is, of course, a question worth asking. Keep in mind, each study also has its own requirements that a person must meet to take part.

Although clinical trials are now done in many different settings, the same rules are in place to protect patients.

What Are the 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 pros and cons 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 are exploratory studies that often use only a few small doses of a new drug in a few patients. 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. The patients in these studies might need extra tests such as biopsies, scans, and blood samples as part of the study process.

The biggest difference between phase 0 and the later phases of clinical trials is that there's almost no chance the volunteer will benefit by taking part in a phase 0 trial – the benefit will be for other people in the future. Because drug doses are low, there's also less risk to the patient in phase 0 studies compared to phase I studies.

Phase 0 studies help researchers find out whether the drugs do what they're expected

to do. If there are problems with the way the drug is absorbed or acts in the body, this should become clear very quickly in a phase 0 clinical trial. This process may help avoid the delay and expense of finding out years later in phase II or even phase III clinical trials that the drug doesn't act as expected to based on lab studies.

Phase 0 studies aren't used widely, 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. The main reason for doing phase I studies is to find the highest dose of the new treatment that can be given safely without serious side effects. Although the treatment has been tested in lab and animal studies, the side effects in people can't always be predicted. 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 often get a very low dose of the treatment and are watched very closely. If there are only minor side effects, the next few participants may 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.
- The focus in phase I is looking at what the drug does to the body and what the body does with the drug.
- Safety is the main concern at this point. Doctors keep a close eye on the people and watch for any serious side effects. Because of the small numbers of people in phase I studies, rare side effects may not be seen until later.
- Placebos (sham or inactive treatments) are not part of phase I trials.
- These studies usually include a small number of people (typically up to a few dozen).
- Often, people with different types of cancer can take part in the same phase I study.
- These studies are usually done in major cancer centers.
- These studies are not designed to find out if the new treatment works against cancer.

Overall, phase I trials are the ones with 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.

Phase II clinical trials: Does the treatment work?

If a new treatment is found to be reasonably safe in phase I clinical trials, it can then be tested in a phase II clinical trial to find out if it works. The type of benefit or response 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 an extended 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 studies look to see if people getting the new treatment live longer than they would have been expected to without the treatment.

Key points of phase II clinical trials:

- Usually, 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.
- In a phase II clinical trial, all the volunteers usually get the same dose. But some phase II studies randomly assign participants to different treatment groups (much like what's done in phase III trials). These groups may get different doses or get the treatment in different ways to see which provides the best balance of safety and effectiveness.
- No placebo (sham or inactive treatments) is used.
- Phase II studies are often done at major cancer centers, but may also be done in community hospitals or even doctors' offices.

Larger numbers of patients get the treatment in phase II studies, so there's a better chance that less common side effects may be seen. If enough patients benefit from the treatment, and the side effects aren't too bad, the treatment is allowed to go on to a phase III clinical trial. Along with watching for responses, the research team keeps looking for any side effects.

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

Treatments that have been shown to work in phase II studies usually must succeed in one more phase of testing 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 have 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 by community-based oncologists.
- 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.

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

Submission for FDA approval: New drug application (NDA)

In the United States, when phase III clinical trials (or sometimes phase II studies) show a new drug is more effective and/or safer than the current standard treatment, a new drug application (NDA) is submitted to the Food and Drug Administration (FDA) for approval. The FDA then 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 type of illness the drug was tested on. If approved, the new treatment often becomes a standard of care, and newer drugs must often be tested against it before being 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, the full 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 typically the safest type of clinical trial because the treatment has already been studied a lot and might have already been used in 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 enrolling 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 clinical trial. But in phase IV studies you're helping researchers learn more about the treatment and doing a service to future patients.

Making the Decision About Clinical Trials

Should I think about taking part in a clinical trial?

This is one of the toughest questions for many people with cancer. The answer won't be

the same for everyone. When trying to decide, first ask yourself some basic questions:

- Why do I want to take part in a clinical trial?
- What are my goals and expectations if I decide to take part? How realistic are these?
- How sure are my doctors about what my future holds if I decide to participate (or not to participate?)
- Have I considered the chance of benefits versus the risks?
- Have I considered other possible factors, such as travel, time, and money?
- Have I considered my other possible options?

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

Risk versus benefit

Each clinical trial offers its own opportunities and risks, but most have some things in common.

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 condition in the future by helping to advance cancer research.
- You could have access to treatment that's not otherwise available, which might be safer or work better than current treatment options.
- You may increase the total number of treatment options available to you, even if you haven't yet had all of the standard treatments.
- You may feel you have more control over your situation and are taking a more active role in your health care.
- You'll probably get more attention from your cancer care team and more careful monitoring of your condition and the possible side effects of treatment if you take part in a clinical trial.
- Some study sponsors may pay for part or all of your medical care and other expenses during the study. (This isn't true for all clinical trials. Be sure you know who's expected to pay for your care before you enroll in the study.)

Some of the possible downsides of being in a study can include:

- The new treatment may have unknown side effects or other risks, which may or may not be worse than those from existing treatments. This is especially true of early phase trials.
- As with other forms of therapy, the new treatment may not work for you even if it helps others.
- There may be inconveniences such as more frequent office visits and testing, as well as time and travel commitments.
- 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 (although this information is available if needed for your safety).
- Insurers may not cover all of the costs of taking part in a clinical trial, but they usually cover the costs of what would normally be standard care. Be sure to talk to your insurance provider and someone involved with the study before you decide to take part, so you know what you may have to pay for.

Common concerns about clinical trials

Most people have some concerns about taking part in a clinical trial, often because they're not really sure what it will mean for them. Taking the time to get as much information as you need before you decide is the best way to be sure that you make the choice that's right for you.

Will there be risks?

Yes, all clinical trials have risks. But any medical test, drug, or procedure has risks. The risk may be greater in a clinical trial because any new treatment has more unknowns. This is especially true of [phase I and II clinical trials](#), where the treatment has been studied in fewer people.

Perhaps a more important question is whether 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 realistic about what this chance is. Ask your doctor to give you an idea of what the possible benefits are, and exactly what benefit is likely for you.

With this in mind, you can make a more informed decision. 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?”

There’s no denying that the ultimate purpose of a clinical trial is to answer a medical question. People who take part in clinical trials may need to do certain things or have certain tests done to stay in the study.

But this doesn’t mean that you won’t get excellent, compassionate care while in the study. In fact, most people enrolled in clinical trials appreciate 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 comfortable with your decision.

Will I get a placebo?

Most cancer clinical trials do not use [placebos](#)¹ unless they are given along with an active drug. It’s unethical to give someone a placebo if it would deny the person a chance to get a drug that’s known to work.

Unfortunately, there are some types of cancer for which there are no proven effective treatments. 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 standard of care already being used.

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

Not for studies that are *randomized*. This means that each person who takes part in the study gets assigned randomly to either the treatment group or the control group. Randomization is used to help reduce the chance that one group will be different from the other when they go into the study, which could affect outcomes. This is especially helpful to make sure that the groups have people in similar states of health, so the results are not skewed in favor of one group. If people were allowed to choose which treatment they got, the study results might not be as accurate. For example, people who were sicker might tend to choose one treatment over the other. If the new treatment was then found not to work as well, doctors couldn’t be sure if this was because the treatment wasn’t as good or because it was tested in sicker people.

Often people have a 50:50 chance of ending up in one group or the other. In some cases, the study may allow for a different ratio, such as 2 out of 3 people getting the new treatment and only 1 out of 3 getting the standard treatment.

Some people find the concept of randomized studies distressing, since neither the patient nor the doctor can choose which group the patient is in. This can be especially true if a study is looking at 2 totally different treatments and a person sees one as 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 form of 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 doctors will be biased in their evaluations of the patients' outcomes. These controls help make the study results more reliable.

Will my information be kept confidential?

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

Medical information that's important for the study, such as test results, is usually put on special forms and into computer databases. This is then given to the people who will analyze the study results. Your information is assigned a number or code – your name isn't on the forms or in the study database. Sometimes, members from the research team or from the Food and Drug Administration might need to look at your medical records to be sure the information they were given is correct. But your personal information isn't given to them and is never used in any published study results.

Questions to ask before joining a clinical trial

Each clinical trial is unique, with its own possible benefits and risks. Before you decide to take part in one, you may want answers to these questions. Some people take notes, record discussions, or bring a friend with them to help recall the answers and think of other questions:

- Why is this study being done?
- What's likely to happen if I decide to take part or decide not to take part in the study?
- Will the researchers work with my cancer doctor? Who will be in charge of my care?
- Who will I contact if I have problems, questions, or concerns?
- What are my other options (standard treatments, other studies)? What are their pros and cons?
- How much experience do you have with this particular treatment? With clinical trials in general?
- What were the results in earlier studies of this treatment? How likely are they to apply to me?
- What kinds of treatments and tests would I need to have in this study? How often are they done?
- Will this require extra time or travel on my part?
- How could the study treatment affect my daily life?
- What side effects might I expect from the study treatment? Are there other risks? (Keep in mind that there can also be side effects from standard treatments and from the disease itself.)
- How will we know if the treatment is working?
- Will I have to be in the hospital for any parts of the study? If so, how often, for how long, and who will pay for it?
- Will I still be seeing my regular cancer doctor? Who will be in charge of my care during the study?
- Will I have to pay for anything? Will any of the treatment be free? 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 study? How long will the study last?
- Are there reasons I would be removed from the study? Are there reasons the study might be stopped early?
- Is long-term follow-up care part of the study? What would it involve?
- If the treatment is working for me, can I keep getting it even after the study ends?
- Can I talk to other patients already taking part in the study?
- Will I be able to find out about the results of the study?
- How long do I have to make this decision?

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 making a decision that's right for you. Also, getting a second opinion from a doctor who's not part of the study may help you decide if a certain study is the best one for you.

How Are Clinical Trial Participants Protected?

Several levels of safeguards are in place to help protect the 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.

Three basic principles, as outlined in the Belmont Report from the late 1970s, provide the basis for research involving humans:

- **Respect for persons:** Recognizing that all people should be respected and have the right to choose what treatments they receive
- **Beneficence:** Protecting people from harm by maximizing benefits and minimizing risks
- **Justice:** Trying to ensure that all people share the benefits and burdens of research equally

These principles are upheld by individuals and groups at the sites conducting research, and also by government agencies charged with overseeing clinical trials. A very important part of patient protection is the informed consent process, which is described in detail in [What's It Like to Be in a Clinical Trial?](#)

Safeguards in institutions

Centers conducting clinical trials have committees that review all potential and ongoing clinical trials to protect the people in the studies. These reviews are required for all federally funded clinical trials, but even privately-sponsored studies must be reviewed.

Institutional review boards (IRBs)

Institutional review boards (IRBs) are groups of people responsible for protecting the welfare of the people who take part in the study and making sure that studies comply with federal laws. The boards are often made up of a mix of medical experts (like doctors and nurses), scientists, and non-medical people. Many institutions have their own IRBs, but some smaller centers may use larger, “central” IRBs. The federal Office of Human Research Protections (OHRP; see below) oversees the activities of IRBs.

Researchers who want to start a study must first submit the study protocol (the plan that describes the study in detail) to the IRB for review. The IRB must decide if the study is acceptable on medical, ethical, and legal grounds. In other words, does the study address a worthwhile question, and is it doing so in a way that ensures the safety of those taking part as much as possible?

One of the most important jobs of an IRB is to make sure the [informed consent](#)¹ form that people entering the study must sign is accurate, complete, and easy to understand. Once a study begins, the IRB also follows its progress regularly to look for potential problems.

If you take part in a clinical trial, you can contact the study’s IRB directly with any questions or concerns regarding safety.

Data safety monitoring boards (DSMBs)

Data safety monitoring boards (DSMBs) are used for [²phase III](#) (and some earlier phase) studies. They are committees made up of doctors and other scientists not involved in the study. Their job is to look at study statistics. They monitor the results of the clinical trial at different time points and can stop a study early (before all of the intended participants have been enrolled or before the study has been completed) if:

- It becomes clear that the new treatment is much more (or much less) effective, so as to allow all study participants to get the better treatment
- Safety concerns arise (such as risks of the new treatment clearly outweighing the benefits), so that no more people are exposed to possible harm

The clinical investigator

The clinical investigator is in charge of all aspects of a particular study. Most often the clinical investigator is a doctor; in some settings this person is called the *principal investigator*, or PI. Ultimately, the responsibility for patient safety in a clinical trial lies with the clinical investigator. Part of this responsibility is letting the study sponsor know

right away when serious side effects occur.

Many clinical investigators have years of experience in running clinical trials. Their credentials are submitted to the FDA along with the [investigational new drug application](#) before the study is approved.

Government agencies

Several government agencies play roles in ensuring that all research is conducted with patient safety in mind. These include:

Office of Human Research Protections

In 2000, the Office of Human Research Protections (OHRP) was set up as the government's main guardian of people's safety and welfare in clinical trials. It enforces the rules regarding the [informed consent](#)³ process, institutional review boards (IRBs), and the participation of people with special needs in clinical trials, such as children and those with mental disabilities. It can suspend research activities until identified problems are corrected.

The OHRP also educates research centers and individuals to help them comply with current clinical trials standards.

Food and Drug Administration

The Food and Drug Administration (FDA) has the final say about whether or not a new treatment is approved to be given to patients. Once phase III clinical trials on a new treatment are completed, the FDA reviews the information and decides if it's safe and effective enough to be approved.

But the FDA's role in many clinical trials starts long before this. Any sponsor seeking approval for a new treatment must submit all study protocols to the FDA before the clinical trials are allowed to begin.

The FDA also inspects (audits) sites conducting clinical trials, especially if there's reason to think they're not following proper procedures. If serious problems are found, the FDA can forbid a particular site or doctor from doing any further research.

But the authority of the FDA is not absolute. Clinical trials that study treatments that are already on the market are not subject to the same FDA regulations (although many are still done in much the same way). And substances considered to be "[dietary](#)

[supplements](#)⁴” do not need FDA approval to be sold in the first place. (Dietary supplement makers aren’t required to prove that their products are safe or effective, so they usually don’t bother to conduct clinical trials.)

National Cancer Institute

The [National Cancer Institute \(NCI\)](#)⁵, part of the National Institutes of Health (NIH), sponsors many of the cancer clinical trials going on at any one time, including those done by cooperative groups. Proposals for such studies must be approved by the NCI before funding is granted. The NCI also regularly audits each site involved in NCI-sponsored research.

How Do I Find a Clinical Trial That’s Right for Me?

There are many ways to find out about clinical trials. Most people who take part in clinical trials do so after hearing about them from their doctors. But you don’t have to wait for your doctor to recommend a clinical trial. Many people with cancer actively look for clinical trials online or in other places, hoping to find more options for treatment.

Where to get information about current clinical trials

At this time there’s no one source to find out about all of the cancer clinical trials enrolling patients. But there are several resources you should know about. There are 2 main types: clinical trials lists and clinical trials matching services.

Clinical trial lists

These sources give you the names and descriptions of clinical trials of new treatments. If there’s a certain study you’re interested in, you’ll probably be able to find it on a list. Lists will often include a description of each study, the criteria for patient eligibility, and a contact person. If you (or your cancer care providers) are willing and able to read through descriptions of all the studies listed for your cancer type, then a list may be all you need. Some organizations provide services that can help you narrow the list a little, according to the kind of treatment you’re looking for (chemotherapy, immunotherapy,

radiation therapy, etc.) and the stage of your cancer.

Sources for clinical trials lists

The **National Cancer Institute (NCI)** sponsors most government-funded cancer clinical trials. The NCI has a list of active studies (those currently enrolling patients), as well as some privately funded studies. You can find the list on their website at www.cancer.gov/clinicaltrials/1 or by calling 1-800-4-CANCER (1-800-422-6237). You can search the list by the type and stage of cancer, by the type of study (for example, treatment or prevention), or by zip code.

The **National Institutes of Health (NIH)** has an even larger database of clinical trials at www.clinicaltrials.gov2, but not all of these are cancer studies.

CenterWatchSM (www.centerwatch.com3) is a publishing and information services company that keeps a list of both industry-sponsored and government-funded clinical trials for cancer and other diseases. You can search their list by things like location, cancer type, or drug name.

Private companies, such as pharmaceutical or biotechnology firms, may list the studies they're sponsoring on their websites or offer toll-free numbers so you can call and ask about them. Some of them also offer matching systems for the studies they sponsor. This can be helpful if you're interested in research on a particular experimental treatment and know which company is developing it.

Clinical trials matching services

Several organizations have developed computer-based systems to match patients with studies they may be eligible for. These services are often offered online.

Each may differ somewhat in how it works. Some of the services let you search for clinical trials without registering at the site. If you have to register, they usually assure you that your information will be kept confidential. Either way, you'll probably have to enter certain details, such as the type of cancer, the cancer stage, and any previous treatments you've had. Using this information, matching services can find clinical trials you might be eligible for, and save you the time and effort of reading descriptions of studies that don't apply to you. Some services also let you subscribe to mailing lists so that you know when new studies open up.

They're usually free to users, but most clinical trial matching services get paid for listing studies or get a finder's fee when someone enrolls. It's important to know this because it may lead to some differences in the way they rank the studies, or the order in which

they present the studies to you.

How to choose a clinical trials matching service

Because each of these services works a little differently, be sure you understand how the service you're looking at operates. Here are some questions to ask. Note that the answers don't necessarily mean that the service isn't worth using; it's just information you may want to have before you decide to use them.

- Is there a fee for using the service?
- Do I have to register to use the service?
- Does the service keep my information confidential?
- Where does the service get its list of clinical trials?
- Does the service rank the studies in any particular order? Is this based on fees they get?
- Can I contact the service online or by phone?

Sources for clinical trials matching services

EmergingMed provides a free, confidential matching and referral service for cancer patients looking for clinical trials at www.emergingmed.com⁴, or you can call 1-877-601-8601.

What you need to know about the clinical trials you find

The study protocol

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

The clinical trial lists available online often include summaries of these protocols, highlighting some key points. Research team members may also have protocol summaries or other information about the study they can share with you.

A protocol contains the following information:

- Why the study is being done (including the goals of the study)
- Information about the treatment being tested (such as names and doses of drugs to be used in the study), often including results of studies done before

- The phase of the study and how many people will be in it
- Who is eligible for the study
- How the treatment is to be given
- What tests will be done during the study and how often they'll be done
- Other information that will be collected on participants
- How long the study will last

Actual study protocols can be very long, 100 pages or more, and they can be very technical. Because they aren't written with patients in mind, making sense of them isn't always easy. Often, the most important information for patients looking for studies is the eligibility criteria (see below) and any information available on the new treatment.

Eligibility, or inclusion and exclusion criteria

All clinical trials have guidelines about who can be in them. Anyone who wants to take part must fit the guidelines. For instance, some studies are looking for volunteers with a certain type of cancer, or a certain stage of disease, while others are looking for people who have just been diagnosed and haven't had any cancer treatment yet. The factors that allow a person to sign up for a study are called *inclusion criteria*. To take part in the study, a person has to meet these criteria.

There are also factors that can exclude a person from each study. For example, a study may be looking for people of a certain age, so people older and younger would not be able to take part. Having certain medical conditions may mean that you can't take part in a study, as can taking certain drugs. Factors that disqualify people from taking part are called *exclusion criteria*. These criteria are often used to be sure that the people in the study can safely take part.

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.

Advertisements and clinical trial lists may not contain all of a study's eligibility criteria. If you've found a study you think you might qualify for, you can usually contact someone involved with the study to get a full list of the criteria.

I think I'm eligible. Now what?

Once you've found a study and confirmed you're eligible for it, deciding if it's the right one for you can still be hard. There may even be more than one study that looks promising. Again, it's important to learn as much as you can.

Talk with someone connected to the study. This could be the clinical investigator 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 meet eligibility criteria before they get into a study. They also make sure that the study protocol is followed for each patient. They often serve as a link between study patients and their doctors.

Both PIs and research coordinators should be able to answer your questions about the study. They can give you answers about their particular clinical trial, but they won't be helpful in discussing 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.

If you haven't done so already, talk to your doctor about the clinical trials you're looking at. Bring in whatever information you can, 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 considering. This discussion can take some time, so you might need to make a special appointment to allow your doctor enough time to look over the information.

You might also want to get a second opinion from a doctor not connected to the studies 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 more promising.

If you have access to the Internet, 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, as well as whether any results are available. If this is hard for you, have

someone close to you help or do it for you.

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

What about cost? Will my insurance cover it?

It's important to get insurance and cost questions answered before deciding to take part in a clinical trial.

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

The Affordable Care Act requires that newer health insurance plans cover the routine costs of care for people who are in approved clinical trials. 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 that treat cancer or other life-threatening diseases, unless the insurance plan is "grandfathered." (Grandfathered plans are any plan or coverage that a person was enrolled in on or before March 23, 2010.)

Clinical trial-related "routine patient costs" that health insurers must cover includes "all items and services consistent with the coverage provided in the plan (or coverage) that is typically covered for a qualified individual who is not enrolled in a clinical trial." This includes things like hospital visits, imaging or lab tests, and medicines.

The law requires 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 study 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 exempt from the IND application

According to the new health care law, insurers do **not** need to pay for:

- The treatment, device, or service that's being studied and is usually covered by the trial's sponsor
- Items and services only needed for data collection and analysis and are not used in direct patient care
- Any service that's clearly not in line with widely accepted and established standards of care for a certain diagnosis

States may require more of their health insurers than this, but these requirements are becoming the minimum. To find out whether your state has special laws about insurance coverage of clinical trials, you can contact your state Health Insurance Commission office, or visit the American Society for Clinical Oncology (ASCO) website at www.asco.org/research-progress/clinical-trials/insurance-coverage-clinical-trials.

Medicare

If you have [Medicare](#)⁵, 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 currently are not covered by Medicare. If you're not sure whether your trial meets all of 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

Find out what your insurer will cover before you enroll in a clinical trial. Other trials may be covered, so be sure to ask about other clinical trials before you start one that may not be covered.

Gather as much information as you can about the study and contact your insurance provider to find out about coverage. 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."

Study sponsors are often eager to recruit eligible patients for their clinical trials, and they may be willing to cover some costs your insurance does not. If needed, ask your doctor or the research coordinator to contact the study sponsor on your behalf.

What's It Like to Be in a Clinical Trial?

Having an idea of what you can expect if you take part in a clinical trial can help relieve some of your concerns. Here's an overview of what might happen.

Informed consent or informed permission

The first thing you'll need to do is give your [informed consent](#)¹ to take part in the clinical trial.

The people running the clinical trial must get your written, informed consent before you take part in any way – even before you have any tests done to see if you're eligible for the study. In the informed consent process, the researchers (doctors or nurses) will explain the details of the study to you and answer your questions and concerns. If you're looking at a study for your child, this process is very much the same but may be called *informed permission*.

You will then be given a written consent form to sign. 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, these children's rights are very much like those of adults, including the right to leave the study anytime they want. Consent forms are not all the same, but they should include the following:

- The reason for the study (what the researchers hope to find out)
- Who is eligible to take part in the study
- What's known about the new type of treatment
- The possible risks and benefits of the new treatment (based on what's known so far)
- 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 are involved
- Who must pay for the costs of the clinical trial (tests, doctor's visits, etc.) and for the

- 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 voluntary nature of the study and your right to leave the study at any time without fear of affecting the care that you would normally get outside the study
- Contact information in case you have questions at any time before and during 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 seem confusing. You may want to bring someone along with you to help make sure all your concerns are addressed.

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 into making a decision. Take the consent form home with you if you need to. Ask trusted family members and friends what they think. Some people may want to get a [second opinion](#)² about the study from another doctor, too.

Taking part in the study

Once you've signed the consent form, you'll be ready to take part in the study. You'll probably need to have [blood tests](#)³ and/or [imaging tests](#)⁴ done before you start treatment (if you haven't had them recently). A full medical history is taken and a physical exam is usually done. These are needed before you start the study to be sure that you meet the eligibility criteria and to help ensure your safety.

As mentioned earlier, the participants are often treated much the same way as other patients who aren't in a clinical trial.

You may have tests done more often to find out how well the treatment is going and to look at how you're doing. You'll likely get more attention as a study participant than you would otherwise. 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 complications may not fully be known, it's very important to let the research team know about anything out of the ordinary. They can then decide if symptoms you're having are related to the study, 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 enter the study that taking part in the study is always voluntary. This is an important point. You (or your child) have the right to leave the study at any time, for any reason. Your doctor will still take care of you to the best of his or her ability.

You may quit taking part in the study at any time and for any number of reasons:

- You complete treatment on the study
- The treatment doesn't seem to be working for you
- You have serious side effects while in the study
- The study itself is stopped early because the treatment either has proven to work, has proven to not work as well as the standard treatment, or has been found to be too harmful
- You decide to leave the study

No matter when or why you leave the study, the researchers may ask to follow up with you from time to time to see how you are doing. This can give them important information and can also help ensure your safety, even though you're no longer part of the study.

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

Some people may be too sick or have other problems that keep them from taking part in clinical trials, but most people will probably be eligible for some type of study. This is true even if they've had many different treatments already.

Some people may be interested in a certain treatment that's only available in clinical trials, but they don't meet the eligibility criteria outlined for the studies. 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 study's clinical investigator, who may consult with others involved in the study about the request. If entered in the study, the person is treated according to the study protocol (the same tests, doctor's visits, follow-up, etc.), but the results from that person 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](#)⁵.

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