Clinical Trials

Improving the Care of People Living With Cancer
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The CancerCare Connect Booklet Series offers up-to-date, easy-to-read information on the latest treatments, managing side effects and coping with cancer.

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Founded in 1944, CancerCare® is the leading national organization providing free, professional support services and information to help people manage the emotional, practical and financial challenges of cancer. Our comprehensive services include counseling and support groups over the phone, online and in person, educational workshops, publications and financial and co-payment assistance. All CancerCare services are provided by oncology social workers and world-leading cancer experts.

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Clinical trials are an important option in cancer care.

Cancer clinical trials are research studies that involve people with cancer. The goal of these studies is to find better ways to diagnose, treat, and prevent cancer. The results of the trials also provide information about how to reduce the risk of cancer in people who have not been diagnosed.

Clinical trials may provide an opportunity for patients to access the latest in cancer care and help identify new therapies for people with cancer. Patients are encouraged to discuss their treatment options, including whether a clinical trial is a viable option, with their health care team.

Doctors across the country and around the world follow treatment guidelines developed from the results of clinical trials so they can deliver the best possible care to their patients. Today there are about 12 million cancer survivors in the United States, mainly because the new therapies developed in clinical trials are helping people with cancer live longer than ever before. That is why it is so important to continue this research.

How Do Clinical Trials Work?

A clinical trial often starts with a scientific idea based on the results of laboratory research. Researchers who come up with these ideas usually work in cancer centers, universities, community clinics, pharmaceutical company labs, or hospitals.

Before any treatment is tested in a clinical trial, many scientists and physicians have studied it to learn how the drug is absorbed into the body, how long it lasts in the body, and whether it should be given by mouth or intravenously (through a needle into a vein). Together, doctors and researchers design the studies and submit an investigational new drug application to the US Food and Drug Administration (FDA). Scientists from the FDA review the procedure, technology, or drug and provide input. Additionally, experts review the trial at many different points during its development and make sure that the clinical trial is designed with the overall goal of improving cancer care for patients.

Clinical trials that test new drugs or other treatments are done in phases. Each phase has a different purpose and helps researchers answer different questions. If a new drug or treatment does not seem promising in the early phases, the research can be stopped.
Advances Made Through Clinical Trials

The standard cancer treatments of today came from yesterday’s clinical trials. The American Society of Clinical Oncology (ASCO), the major organization for cancer specialists, has identified the five most important accomplishments made through clinical trials over the past 50 years of ASCO’s history:

- Chemotherapy cures for Hodgkin lymphoma: 90% of patients with Hodgkin’s disease can now be cured of the disease regardless of whether it is diagnosed early or in an advanced stage
- The human papilloma virus (HPV) vaccine (e.g. Gardasil®, Cervarix®), which can prevent cervical cancer
- A targeted drug (Gleevec® and others) for chronic myelogenous leukemia (CML) that has made CML a chronic, manageable disease for many patients and is used for other cancers as well
- The three-drug regimen (the Einhorn regimen, which includes cisplatin, bleomycin, and vinblastine) that cures almost 100% of men with advanced testicular cancer
- Anti-nausea drugs such as Zofran that have improved the quality of life for people with cancer

All of these discoveries were made through carefully designed clinical trials, making these trials an extremely important option for people with cancer to consider.

Phases of Clinical Trials

In phase I trials, researchers study the safety of a new drug or drug combination or a new dose of a drug. Phase I cancer trials usually only include a small group of 15 to 30 patients, and they may have any tumor type. This type of study looks at safety and any side effects or toxicities that might occur with the treatment. In phase I trials, researchers try to find the lowest dose that will still be effective, and determine which patients are able to tolerate the drug. These trials usually take place in research centers, where patients can be closely monitored.

In phase II trials, the new drug or treatment is given to a larger group of people (but generally fewer than 100) to see if it is effective and to learn more about its safety. Like phase I studies, phase II trials are usually conducted at research centers, such as large teaching hospitals and cancer centers. In some phase II studies, patients may be randomized. This means patients in the trial are selected by chance to get either the new treatment being studied or one that is already being used to treat their type of cancer. In order to avoid influencing the results, patients (and often their doctors) do not know which group they are in. Usually, there are limitations in phase II trials so that the drug can only be given to patients with a specific disease or a particular patient population.

Randomized clinical trials are considered the most reliable way of determining which treatments work best.

In phase III trials, the study drug or treatment being tested is given to an even larger group of people (often as many as 1,000 to 3,000). During this phase, researchers are able to:

- Confirm how well the treatment works against the cancer
• Learn about side effects they might not have seen during earlier phases with fewer patients
• Compare the new treatment with currently used standard treatments
• Collect information that will allow the new drug or treatment to be used safely

In phase III trials, patients are randomized, as described above. In cancer clinical trials, patients are generally not given a placebo (a look-alike pill or liquid that contains no active ingredient). A placebo is only used when there is no standard treatment against which a new treatment can be compared.

Phase III studies are often carried out in the community by local doctors in private practice, community hospitals, or designated cancer centers. Because phase III trials involve large numbers of people, doctors can better understand how a treatment will work in people with many different health issues.

In phase IV trials, researchers study drugs after they have been approved by the FDA. Phase IV trials are designed to learn more about the treatment’s risks and benefits and the best way to use it. These studies help doctors understand how safe and useful the treatment will be over the long term.

There are also studies with very little risk that do not have an active drug or technology to test, such as quality-of-life studies. In these studies, researchers just want to learn more about patients’ quality of life, and the studies can include almost anyone with cancer.

Care Across Phases of Clinical Trials

At different points in time, a patient might be eligible for a phase I, II, or III study. Most often in phase III trials, a patient’s regular cancer care provider continues their care. For phase I and II studies, however, very often they will be treated by groups of doctors, nurses, and support staff who manage the studies within the cancer center. There is a slightly greater potential risk associated with phase I and II studies since less is known about the treatment early on, so patients’ health is carefully monitored in these studies. This means there can sometimes be a transition in the care team from the usual provider to health professionals involved in the study.

There has to be good communication between teams of providers who take care of patients in different phases. Study doctors will do a thorough review of the patient’s previous medical records. They often have very detailed discussions with the previous care provider. If a patient is enrolled in a phase I study located far from where they receive their usual care, communication is very important between the old and new locations, and between the previous health care providers and the team who will be caring for the patient going forward.
“Personalizing” Cancer Treatment

Genes are the blueprint for each cell in the body, including tumor cells. They determine how that cell behaves. Sometimes, when a particular treatment is working well in some patients but not in others, researchers may discover that having—or not having—a certain gene or a variation in a gene may be the reason.

In a new approach to clinical trials, new participants joining the trial whose tumors have the same genetic makeup are more likely to receive the drug. This gets answers more quickly and requires fewer participants to find out whether a drug works.

Types of Clinical Trials

Depending on the goals of the trial, there are several different types to consider:

- **Prevention trials** look for better ways to reduce the risk of cancer in people who have never had it or to prevent the cancer from coming back. These approaches may include medicines, vitamins, vaccines, or lifestyle changes.
- **Screening trials** test the best way to detect cancer.
- **Diagnostic trials** try to find better tests or procedures for diagnosing a particular cancer.
- **Treatment trials** test new treatments, new combinations of drugs, new ways to deliver treatment, or new approaches to surgery or radiation.
- **Quality-of-life trials (or supportive care trials)** explore ways to improve comfort and the quality of life for people with cancer.
What Are the Benefits of Joining a Clinical Trial?

Being in a clinical trial lets you play a more active role in your health care. Your own cancer doctor will still care for you, but in a clinical trial you also receive:

- **Close observation by cancer experts.** Because a clinical trial must follow a strict protocol, or plan, patients participating in clinical trials are very closely observed. This is the only way researchers can be sure that the information they get from the study is accurate and complete.

- **Access to new cancer treatments and diagnostic and therapeutic techniques** (such as a better way of delivering treatment) before they are widely available. Doctors perform clinical trials because they believe the drugs or techniques they are testing could be more effective or safer than the treatments they are using now. But doctors also have to prove it scientifically. Only with this proof will the FDA approve the new treatments or techniques for other people with the same cancers.

As with all medical treatments, there may be risks associated with the treatments used in clinical trials. These risks include side effects and the possibility that the new treatment may not work as well as the researchers had hoped, or in some instances that the treatment causes more adverse effects than were expected.

Before you agree to enter a study, your doctor or nurse will very carefully explain in great detail the possible risks and make sure you understand them. Researchers want patients to be aware that although these new ideas and treatment strategies may contain their best hopes, they are still experimental strategies.

Another thing to consider is that participating in a clinical trial may take more time and attention than you expected. There may be trips to the hospital or clinic where the study is being done, hospital stays, or a complex schedule for taking medications. Always ask your health care team any questions you may have about the clinical trial.

Weighing the risks and benefits of a clinical trial is a very personal decision. There is no right or wrong answer. Only you can decide if a trial is right for you.

Am I Eligible for a Clinical Trial?

Not everyone will be accepted into every trial. When planning a clinical trial, researchers decide on the characteristics of the people they would like to study. Each trial has a different set of characteristics. Some common examples include:

- Age
- Cancer type and stage
- Treatments the person has already undergone
- Other medical concerns, such as diabetes or heart disease

If you think you would like to take part in a clinical trial, ask your doctor or nurse about the trials available for your cancer. You can also search online using the websites in our resource list on page 21, such as www.clinicaltrials.gov. There are toll-free numbers available as well, so you can talk to someone who can guide you.

You can also find a hospital or center near you that has National Cancer Institute or NCI clinical trials. These are Centers of Excellence, made available throughout the country in many locations. Currently there are more than 30 of these facilities.
What Questions Should I Ask?

If you and your health care team have decided that a clinical trial is the right choice for you, there are a number of questions you should ask, including:

- What is the purpose of the study?
- What kinds of tests and treatments are involved?
- How do the possible risks, side effects, and benefits in the study compare with my current treatment?
- How might this study affect my daily life?
- How many visits per week or month will I need to make?
- How long will the study last?
- Is a hospital stay required?
- Who will pay for the treatment? Will the trial, or my insurance, cover all or part of it?
- What will I need to pay for myself?
- Will I be reimbursed for any expenses such as transportation?
- What type of long-term follow-up care is part of this study?
- How will I know that the treatment being studied is working? Will the results of the trial be given to me?
- Who will be in charge of my care?
- Are there other experts I can talk to about this study?
- Can I take the informed consent form home to talk it over with my family or partner?

What Rights and Protections Do I Have in a Clinical Trial?

People who take part in clinical trials have both rights and protections to make sure their privacy and well-being are taken care of. One of the most important protections is called informed consent. This is a process to ensure that the participant understands all aspects of the study, including the risks and benefits. A written consent document is signed by a patient, stating that he or she has entered the trial of his or her own free will.

As a patient in a clinical trial, you always have the right to leave a trial at any time for any reason. There will be no penalty and no harm done; all patients always have that legal and ethical right. Your doctor will also be in communication with the trial, and if it seems that the treatment is not working, the physician and patient together may decide to leave the trial.

Besides informed consent, you will be asked to sign another form called a HIPAA Authorization. HIPAA stands for Health Insurance Portability and Accountability Act. This form allows
doctors to use your health information as part of the report on the study, without using your name or other personal details.

Each research institution, hospital, or cancer center that conducts clinical trials has a committee called an Institutional Review Board (IRB). An IRB includes doctors, nurses, lawyers, and people affected by cancer. The IRB reviews each clinical trial to make sure it is safe and ethical, in order to protect the people who take part. They review the study on a regular basis to be sure it is being carried out properly.

If you have concerns about any part of being in a clinical trial, speak with a patient representative at the institution where the trial is being conducted. The name and contact information for this person are usually included on the informed consent document. If a patient representative is not listed, ask if there is someone else you can speak with, such as a nurse or social worker, to answer any questions you might have.

Languages Other than English

It can be difficult if the patient and health care team members do not speak the same language. Hospitals and cancer centers have professional interpreters available to help with this issue, either in person, or remotely through a phone or video line. The Resources section of this booklet has recommendations for Spanish speakers. There are many trial brochures that have been translated into Spanish. If the patient cannot read the informed consent form in English in order to enroll in a trial, there will be a shorter form presented in Spanish, but the investigator will carefully explain the form with the help of a qualified, certified medical interpreter.

Organizations such as CancerCare® and the American Cancer Society have bilingual, bi-cultural staff who can help with your questions or help you to access experts to help you make decisions about clinical trials in Spanish.
CancerCare® Can Help

When you are being treated for cancer and are thinking about entering a clinical trial, you may have many questions about the process. It’s perfectly normal to feel confused or nervous about clinical trials. But the more you learn about what’s involved and what to expect, the better you’ll feel about your decision.

Help is available to you as you consider your options. It is very important to have a support network when you are diagnosed with cancer, and your health care team, family members, and friends will likely be your main source of support. CancerCare is also here to help and can be a part of that network. All of our services are provided free of charge, including:

Counseling. Often, when people are thinking about enrolling in a clinical trial, they need someone to talk with who will help them sort through the emotional and practical concerns that come up. Oncology social workers provide emotional support, help you find ways to stick with treatment and cope with side effects, and refer you to resources. CancerCare provides free counseling from professional oncology social workers who can help you understand what enrolling in a clinical trial means for you and your family.

Support groups. Talking with other people who have taken part in clinical trials can help reduce the feeling that you are going through it alone. These groups provide reassurance, suggestions, and insight—all in a safe and supportive place where people can share similar concerns. At CancerCare, people with cancer and their families take part in support groups in person, online, or on the telephone. All groups are facilitated by oncology social workers.

Connect Education Workshops. In these workshops, you can hear about the latest treatments and clinical trials directly from leading medical experts in one-hour presentations. You can listen live by telephone or online, and you will have the chance to ask the experts your own questions. You can also download podcasts of past workshops from our website.

Publications. Free booklets and fact sheets from CancerCare provide up-to-date, easy-to-read information about the latest on clinical trials, treatments, managing side effects, and coping with cancer.

Financial help. For those who qualify, CancerCare provides limited financial assistance to help with some treatment-related costs such as transportation and child care.

Referrals to resources. CancerCare can help you learn about other organizations in your community and nationwide that can assist you in finding a clinical trial that is right for you.

To learn more about how we can help, please call our Hopeline at 800-813-HOPE (4673) or visit www.cancercare.org.
Frequently Asked Questions

Q. If I find a clinical trial online, can I just sign up, or do I have to go through my doctor?

A. At www.clinicaltrials.gov, for example, you may get in touch with the contact person listed, but you cannot sign up online. The National Institutes of Health, which operates this website, recommends that you talk with your doctor about the right clinical trial for you.

You should know that all clinical trials have guidelines about who can participate. These qualifications are used to:

- Identify the right patients for the trial.
- Keep all participants safe.
- Make sure that researchers will be able to answer the questions they plan to study.

Your doctor can advise you about whether you qualify for a study and help you contact the researchers involved.

Before you meet with your doctor for that discussion, do your homework by searching online or calling the toll-free numbers listed on page 21. Your doctor may not know about newer studies that are available for you. Some people with cancer have placed their names in databases for future clinical trials. If you let your doctor know that you would like to hear about new clinical trials, he or she can help you stay informed.

Q. I’m in a clinical trial now and have experienced side effects from the drug I’m taking. Do the doctors need to stick with the plan of the clinical trial?

A. All doctors in a clinical trial must follow protocols—that is, the plan for what treatment will be studied and how it will be given to patients. However, it is very important to let your health care team know about any side effects you are having. This is the only way they will be able to help you manage your symptoms so you can feel better. Knowing what side effects the drug causes is also important for research purposes. You are helping yourself and the researchers by being as honest as possible.

Q. I’ve been told I can’t be in some clinical trials because I have high blood pressure. Can trials exclude people with certain medical conditions?

A. Yes, sometimes patients with certain conditions are excluded. The safety of patients in clinical trials comes first. If the researchers believe that high blood pressure or some other medical condition puts a person at risk, they will not enroll the person. However, you should talk to your doctor about your particular case. Each trial is different, and sometimes these decisions are made on a case-by-case basis. So you still might qualify for some clinical trials.

Q. I’m in treatment and doing well. Should I enter a clinical trial?

A. When treatment is going well, a person with cancer may still wonder if he or she should join a clinical trial. Talk with your doctor about whether the treatment you would receive in a clinical trial may improve upon the treatment you’re already getting. Some trials study new treatments that may prevent the return of cancer after your initial treatment is complete. Your doctor may suggest you take part in one of these clinical trials in the future.
Q. I live in a rural area. I can’t convince my doctor to sign me up for a clinical trial. What should I do?

A. Living in a rural area may make it more challenging to get the kind of care that you want. Often, there are not as many cancer specialists (oncologists) in rural areas as there are in a city or the suburbs. But there are other options you can explore. Perhaps there is another doctor in a nearby town where you could take part in a clinical trial.

Many states have at least one comprehensive cancer center where clinical trials are underway. (See our resource list on page 21 for information on finding a center near you.) A comprehensive cancer center is a large hospital that specializes in caring for patients with all types of cancer, conducting clinical trials, and teaching the public about cancer. The National Cancer Institute selects these centers; there are more than 60 in 33 states. These centers may be far from your home, but sometimes the sponsor of the trial or organizations such as CancerCare may be able to help you with some travel expenses.

Q. I’m 72 years old. Can I still take part in a clinical trial?

A. We used to think that because of their age, older adults would not be able to tolerate strong treatments. We now know that a person’s overall health, rather than his or her age, is more important when it comes to clinical trials. Sometimes a clinical trial might be studying only patients in a certain age range. But in general, age alone should not prevent you from entering a trial. Researchers are encouraging older adults to take part in clinical trials so that seniors can receive the treatment benefits. Doctors also want to be sure that the medicines are, in fact, safe and effective for older people with cancer. Clinical trials are the best way to find out.