Clinical Trials:
Improving the Care of People Living with Cancer

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Clinical Trials: Improving the Care of People Living with Cancer

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A clinical trial is a research study designed to evaluate a new treatment approach. Clinical trials are the only way new treatments can emerge that improve the lives of people with cancer.

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

There are many types of cancer clinical trials, including treatment, screening, prevention, symptom management and quality of life. This booklet focuses on cancer treatment trials that evaluate medications (drugs). However, it is important to note that other treatment methods—including surgery and radiation—are also evaluated in clinical trials.

Many clinical trials test an already-approved drug, for use in a different way or to treat a different disease. Other trials evaluate a drug (or a combination of drugs) not previously given to humans, to make sure the approach is safe and will provide benefit to patients.

Many times, researchers and doctors study a new drug in the laboratory before a human clinical trial is designed and proposed. Among other things, they want to know how the drug is absorbed into the body, how long it remains in the body, the maximum tolerated dose and whether it should be given by mouth or intravenously (through a needle into a vein).

If supported by the results of the laboratory study, researchers and doctors create a Clinical Protocol, a detailed blueprint of every aspect of the trial. The clinical trial sponsor then submits an Investigational New Drug (IND) application and the Clinical Protocol to the U.S. Food and Drug Administration (FDA). The FDA has 30 days to review the IND and Clinical Protocol to both provide input and to assure that the trial will safely and ethically evaluate the drug and that the trial participants will not be subjected to unreasonable risk. (A clinical trial sponsor is a person, group, company, institution, organization or government agency that oversees or pays for a clinical trial, and collects and analyzes the data.)

When discussing your treatment options with your oncologist, ask if a clinical trial might be right for you. Clinical trials are routinely integrated into the treatment plan for people with cancer.

Here a few things to consider:

- Most clinical trials are designed to test a new treatment approach to find out whether the approach is safe and has any added benefit.
- Often, people who take part in clinical trials gain access to—and potential benefit from—new treatments that are not otherwise available.
- All clinical trials have eligibility requirements, and you may or may not qualify to participate in any specific trial.
- Before you participate in a clinical trial, you will be fully informed of the risks and benefits of the trial, including any possible side effects.
- You have the right to stop taking part in a clinical trial at any time for any reason.

The Resource section of this booklet provides websites that allow you and your doctor to search for a clinical trial that might be right for your individual circumstances, including your type of cancer and your geographic location.

Clinical Trial Phases

Clinical trials that evaluate new treatment approaches are done in phases. Each phase has a different purpose, helping researchers to answer different questions. If a new drug or treatment approach does not seem promising in an early-phase trial, the research can be stopped. Every clinical trial, regardless of its phase, has a Principal Investigator (often a medical doctor), who is responsible for leading the trial.

Clinical trials, including those focused on cancer, follow these established phases:

Phase I. In phase I trials, researchers study the safety of a new drug (or drug combination) or a new dose of a currently-approved drug. Researchers try to find the lowest dose that will still be effective, determine the tolerability of the drug and study side effects that might occur. These trials usually include a small number of participants and take place in research centers, where the participants can be closely monitored.

Phase II. The purpose of this phase is to determine if the treatment is effective and to learn more about its safety. In some phase II trials, participants are "randomized," meaning they are selected by chance to either receive the treatment being studied, or one that is already being used to treat their type of cancer. Phase II trials typically involve many more patients than a phase I trial and tend to be conducted in research centers. It is important to note that in cancer clinical trials, participants are generally not given a placebo, which is a 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. In this phase, the treatment being tested is given to hundreds or even thousands of participants, who are often randomized as in phase II trials. There are a number of purposes of this phase:

- To confirm how well the treatment works against the cancer.
- To learn about side effects that might not have been seen during earlier phases.
- To compare the new treatment approach with current approaches.
- To collect information that will allow the new treatment approach to be used safely.

Because of the large number of participants, phase III studies are often carried out in multiple sites, including by doctors in private practice, in community hospitals and in designated cancer centers.

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

The Value of Cancer-Focused Clinical Trials

Clinical trials help identify new cancer therapies and provide people diagnosed with cancer an opportunity to access the latest treatment approaches. These trials are the standard by which we measure the worth of new treatments and the quality of life of people as they receive those treatments.

Many trials include an assessment of the genetic or molecular characteristics of a person's cancer that could be linked to the success of a specific type of treatment.

The American Society of Clinical Oncology (ASCO), a leading professional organization representing physicians who care for people with cancer, has identified the five most important accomplishments made through clinical trials over the last 50 years:

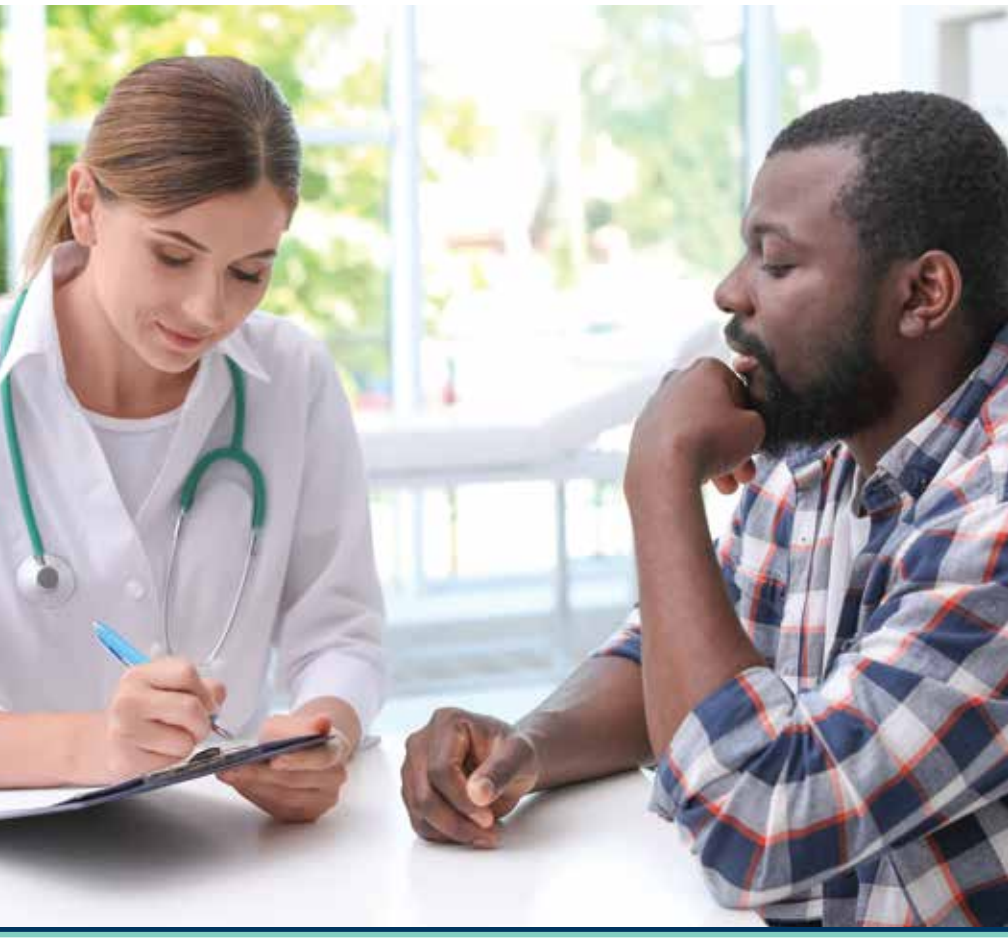
- As a result of chemotherapy advancements, 90 percent of Hodgkin lymphoma cases can now be cured regardless of whether they are diagnosed early or at an advanced stage.
- Cervical cancer can be prevented by the human papilloma virus (HPV) vaccine.
- Imatinib, a targeted therapy, results in long-term remission of most cases of chronic myelogenous leukemia (CML) and is used in the treatment of many other types of cancer.
- A three-drug regimen (the chemotherapies cisplatin and vinblastine and the anti-tumor antibiotic bleomycin) cures almost 100 percent of men with advanced testicular cancer.
- Anti-nausea drugs that have been developed, evaluated and approved by the FDA have improved the quality of life for people with cancer.

If you have been diagnosed with cancer, there are a number of benefits associated with clinical trial participation, including:

- **Access to promising new cancer treatments.** Clinical trials often evaluate cutting-edge therapies that are not available outside of the clinical trial setting, providing you with a treatment option that may be more effective than your current therapy.
- **The chance to play a more active role in your health care.** Participating in a clinical trial is educational, informative and allows you to gain a greater understanding of your cancer and its treatment. It also has the potential to increase the control you have over your situation, which can lead to reduced stress levels and a better quality of life.
- **Close observation by cancer experts.** Although your oncologist will likely still be responsible for providing your overall cancer care, clinical trial participants are closely observed by the trial team. In part, this is because a clinical trial must follow a strict protocol (plan) and researchers need to be sure that the information they get from the trial is accurate and complete.
- **Contributing to cancer research.** People who participate in clinical trials are vital to advancing medical care for people with cancer. The information gathered in clinical trials adds to scientific knowledge and can potentially lead to the approval of new treatment approaches.

As with all drugs, there may be risks associated with drugs used in clinical trials. These risks include side effects and the possibility that the treatment may not work as well as the researchers had hoped. Before you agree to enter a trial, a member of your health care team will carefully explain these potential risks.

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



Rights and Protections

People who take part in clinical trials have specific rights and protections to ensure their privacy and well-being.

Data and Safety Monitoring Board. Randomized clinical trials are commonly overseen by a Data and Safety Monitoring Board (DSMB), an independent group of experts who monitor patient safety and treatment efficacy (effectiveness) data. DSMBs are composed of experts in medicine, ethics and biostatistics.

Institutional Review Board. Each research institution, hospital or cancer center that conducts clinical trials has a committee called an Institutional Review Board (IRB), which includes doctors, nurses, lawyers and people affected by cancer. The IRB reviews each clinical trial to make sure it is safe and ethical. Members of the IRB also review the study on a regular basis to make sure it's being carried out as designed.

Informed Consent. Clinical trial participants are required to review and sign an Informed Consent form, which outlines what a "reasonable person" would want to know to make an informed decision about whether or not to participate in the trial. Written in lay (plain) language, the Informed Consent form includes:

- The nature of the trial.
- Why the person is a candidate for participation in the trial.
- The risks and benefits of participation.
- Alternatives to participation.
- Their rights as clinical trial participants.

By signing the Informed Consent form, the participant is attesting that they understand what is outlined and are entering the trial of their own free will.

HIPAA Authorization. Participants also sign a HIPAA (Health Insurance Portability and Accountability Act) form, which allows doctors to use their health information as part of the report on the study, but without including their name or other information that could identify them.

Patient Representatives. People who have questions or concerns while participating in a clinical trial can speak with a patient representative at the institution where the trial is being conducted. The name and contact information for this person is usually included on the Informed Consent form. If a patient representative is not listed, participants can ask if there is someone else at the trial site they can speak with, such as a nurse or social worker.

The right to leave. Participants always have the legal and ethical right to leave a trial at any time, for any reason. The decision to leave can be made by the participant alone; however, it may be beneficial to the participant to make the decision in collaboration with their oncologist or another member of their health care team.

Questions To Ask

Here is a list of questions to ask your oncologist if you are considering participation in a specific clinical trial. They may refer you to a trial representative (e.g. a Patient Representative or Case Manager) for some of these questions, but it's best to begin the discussion with your oncologist.

Initial Questions

- What is the purpose of the trial?
- What are the eligibility requirements of the trial?
- What kinds of tests and treatments are involved?
- How long will the study last?
- Will you and other members of my health care team still be in charge of or involved in my care?
- Are there alternative treatments for me, other than what is being offered in the clinical trial?

Quality of Life Questions

- Is a hospital stay required?
- How many visits per week or month will I need to make?
- How do the possible risks, side effects and benefits of the study compare with my current treatment?
- Is long-term follow-up care part of this study?
- How will I know that the treatment being studied is working?

Logistical Questions

- Are there other drugs or supplements that I will be required to take during the trial?
- Are there drugs or supplements that I cannot take during the trial?
- Who will pay for the treatment? Will the trial, or my insurance, cover all or part of it?
- Will I be reimbursed for any expenses, such as transportation?
- Will the results of the trial be given to me?

Communicating With Your Health Care Team

As you manage your cancer treatment, including considering clinical trial participation, it's important to remember that you are a consumer of health care. The best way to make decisions about health care is to educate yourself about your diagnosis and get to know the members of your health care team, including doctors, nurse practitioners, physician assistants, nurses, dietitians, social workers and patient navigators.

Here are some tips for improving communication with your health care team:

Start a health care journal. Having a health care journal or notebook (either on paper or in a digital format) will allow you to keep all of your health information in one place. You may want to write down the names and contact information of the members of your health care team, as well as any questions for your doctor.

Bring someone with you to your appointments or have them be present during telehealth sessions. Even if you have a journal and a prepared list of questions or concerns, it's always helpful to have support during your appointments. The other person can serve as a second set of ears. They may also think of questions to ask your doctor or remember details about your symptoms or treatment that you may have forgotten.

Write down your doctor's answers. Taking notes will help you remember your doctor's responses, advice and instructions. You can also ask the person who accompanies you to take notes for you, either in your journal or on a tablet or smartphone.

Record your visit if your doctor allows it. Recording the conversation with your doctor gives you a chance to hear specific information again or share it with family members or friends.

Incorporate other health care professionals into your team. Your medical oncologist is an essential member of your health care team, but there are other health care professionals who can help you manage your diagnosis and treatment:

- Your primary care physician should be kept updated about your cancer treatment and any test results.
- Your local pharmacist is a great source of knowledge about the medications you are taking. Have all of your prescriptions filled at the same pharmacy to avoid the possibility of harmful drug interactions.
- Make sure your oncologist knows of any other medical conditions you have or any pain you are Remember, there is no such thing as over-communication.

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Cancer*Care*'s Free Support Services and Programs

When people are thinking about enrolling in a clinical trial, they often need someone to talk with who can help them sort through all of their concerns. That's where Cancer*Care* can help.

Cancer*Care* is a national nonprofit organization providing free, professional services to anyone affected by cancer. Our licensed oncology social workers can help you understand the clinical trial process, provide guidance on coping with potential treatment side effects once the trial begins, and refer you to other valuable informational resources, including:

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 environment. At Cancer*Care*, people with cancer and their families take part in support groups in person, online or on the telephone. All groups are facilitated by licensed oncology social workers.

Connect Education Workshops. Delivered by leading medical experts, these one-hour presentations cover a number of valuable topics for people coping with cancer, including updates on FDA-approved treatments for specific cancer types and research currently being conducted in clinical trials. You can listen live by telephone or online, and you'll have the chance to ask the experts your own questions. You can also download past workshops from our website.

Publications. Free booklets and fact sheets from Cancer*Care* provide up-to-date, easy-to-read information about clinical trials, treatment updates, managing side effects and improving your quality-of-life while coping with cancer.

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

To speak with one of Cancer*Care's* oncology social workers, call us at 800-813-HOPE (4673).

Frequently Asked Questions

Q: I'm in a clinical trial and have experienced side effects from the drug I'm taking. What should I do?

A: All cancer treatments, including those given in a clinical trial, can cause side effects. It's important that you immediately report any side effects that you are experiencing to a doctor or other designated person at the clinical trial site. By doing so, you are helping yourself and contributing significantly to the work of the researchers. It's also important to report these side effects to your health care team so they can help you manage them. Doing so will improve your quality of life while you are participating in the trial.

Q: Can trials exclude people with certain medical conditions?

A: The safety of clinical trial participants comes first. If the researchers believe that a medical condition puts a person at risk, they will exclude that person from the trial. If you have been excluded, talk to your oncologist about other possible options. Each clinical trial has its own set of enrollment standards, so you might qualify for another trial.

Q: What is a quality of life clinical trial?

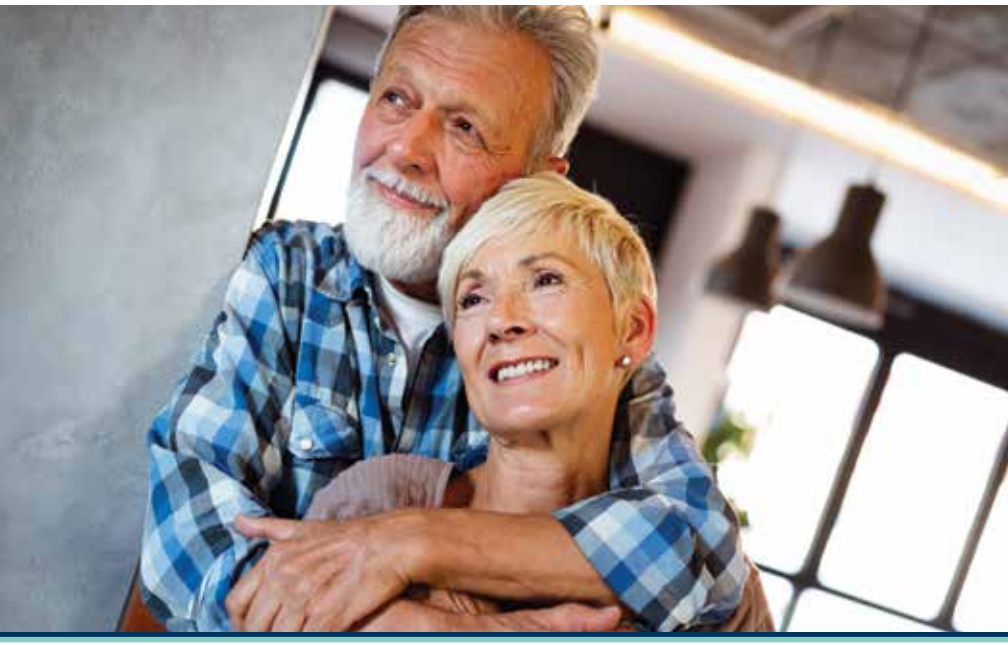
A: Quality of life trials do not evaluate any specific therapy; rather, they address such issues as short and long-term effects of therapy, prevention of pain and issues related to nutrition and stress. These types of trials are often available to people being treated for cancer.

Q: I'm in treatment for my cancer and doing well. Is there any reason for me to participate in a clinical trial?

A: This is a good topic to discuss with your oncologist. There could be a current or upcoming clinical trial for a treatment that may be of additional benefit to you. Also, some clinical trials evaluate treatments that may prevent the return of cancer after your initial treatment is complete. Your oncologist may suggest you take part in one of these trials in the future.

Q: I'm 75 years old. Is there an upper age limit for participating in a clinical trial?

A: In general, age alone does not exclude a person from participating in a clinical trial. Other factors, including overall health and existing medical conditions, are taken into consideration when the decision is made to include or exclude a potential trial participant. As researchers are interested in learning about the safety and effectiveness of the drug or drugs being tested in older people, some trials are designed to focus on people in an older age range.



Resources

CancerCare®
800-813-HOPE (800-813-4673)
www.cancercare.org

American Cancer Society
800-227-2345
www.cancer.org

Cancer.Net
Patient information from the American Society of Clinical Oncology
888-651-3038
www.cancer.net

Cancer Support Community
888-793-9355
www.cancersupportcommunity.org

National Cancer Institute
800-422-6237
www.cancer.gov

CLINICAL TRIALS WEBSITES

ClinicalTrials.gov
www.clinicaltrials.gov

EmergingMed
www.emergingmed.com

National Cancer Institute
www.cancer.gov/research/areas/clinical-trials

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