Taking Part in Clinical Trials

What Cancer Patients Need To Know

NATIONAL INSTITUTES OF HEALTH
National Cancer Institute

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Contents

Purpose of This Booklet

This booklet is for people with cancer, their families, and others who care about them. It is divided into three sections. Each section answers questions many people have about clinical trials:

1. What are clinical trials?
2. What happens in a clinical trial?
3. Should I take part in a clinical trial?

The first two sections provide background information on the important role clinical trials play in improving cancer care. They also explain some of the technical terms you may hear from your doctor or nurse. Words that appear in **bold** on pages 1 to 15 are defined in the Glossary that begins on page 17.

The third section of the booklet is designed to help you answer question 3 for yourself. It raises issues to think about as you decide whether a clinical trial is right for you. For example, what are the pros and cons of being in a clinical trial from the patient's point of view? This section also lists some questions to ask the doctor or nurse about any study you are considering. The resources section on page 15 lists other sources of information about cancer and treatment studies.

This booklet is a part of the patient education program of the National Cancer Institute (NCI). The NCI sponsors, conducts, and oversees clinical trials and other cancer research, and provides research-based information to health professionals, patients, and the public.

What Are Clinical Trials?

Clinical trials, also called cancer treatment or research studies, test new treatments in people with cancer. The goal of this research is to find better ways to treat cancer and help cancer patients. Clinical trials test many types of treatment such as new drugs, new approaches to surgery or radiation therapy, new combinations of treatments, or new methods such as gene therapy.

A clinical trial is one of the final stages of a long and careful cancer research process. The search for new treatments begins in the laboratory, where scientists first develop and test new ideas. If an approach seems promising, the next step may be testing a treatment in animals to see how it affects cancer in a living being and whether it has harmful effects. Of course, treatments that work well in the lab or in animals do not always work well in people. Studies are done with cancer patients to find out whether promising treatments are safe and effective.

Why Are Clinical Trials Important?

Clinical trials are important in two ways.

First, cancer affects us all, whether we have it, care about someone who does, or worry about getting it in the future. Clinical trials contribute to knowledge and progress against cancer. If a new treatment proves effective in a study, it may become a new **standard treatment** that can help many patients. Many of today's most effective standard treatments are based on previous study results. Examples include treatments for breast, colon, rectal, and childhood cancers. Clinical trials may also answer important scientific questions and suggest future research directions. Because of progress made through clinical trials, many people treated for cancer are now living longer.

Second, the patients who take part may be helped personally by the treatment(s) they receive. They get up-to-date care from cancer experts, and they receive either a new treatment being tested or the best available standard treatment for their cancer. Of course, there is no guarantee that a new treatment being tested or a standard treatment will produce good results. New treatments also may have unknown risks. But if a new treatment proves effective or more effective than standard treatment, study patients who receive it may be among the first to benefit. Some patients receive only standard treatment and benefit from it.

In the past, clinical trials were sometimes seen as a last resort for people who had no other treatment choices. Today, patients with common cancers often choose to receive their first treatment in a clinical trial.

What Happens in a Clinical Trial?

In a clinical trial, patients receive treatment and doctors carry out research on how the treatment affects the patients. While clinical trials have risks for the people who take part, each study also takes steps to protect patients.

What Is It Like To Receive Treatment in a Study?

When you take part in a clinical trial, you receive your treatment in a cancer center, hospital, clinic, and/or doctor's office. Doctors, nurses, social workers, and other health professionals may be part of your treatment team. They will follow your progress closely. You may have more tests and doctor visits than you would if you were not taking part in a study. You will follow a treatment plan your doctor prescribes, and you may also have other responsibilities such as keeping a log or filling out forms about your health. Some studies continue to check on patients even after their treatment is over.

How Is the Research Carried Out? How Are Patients Protected?

In clinical trials, both research concerns and patient well-being are important. To help protect patients and produce sound results, research with people is carried out according to strict scientific and ethical principles. These include:

1. Each clinical trial has an action plan (protocol) that explains how it will work.

The study's **investigator,** usually a doctor, prepares an action plan for the study. Known as a **protocol,** this plan explains what will be done in the study and why. It outlines how many people will take part in the study, what medical tests they will receive and how often, and the treatment plan. The same protocol is used by each doctor that takes part.

For patient safety, each protocol must be approved by the organization that sponsors the study (such as the National Cancer Institute) and the **Institutional Review Board (IRB)** at each hospital or other study site. This board, which includes consumers, clergy, and health professionals, reviews the protocol to try to be sure that the research will not expose patients to extreme or unethical risks.

2. Each study enrolls people who are alike in key ways.

Each study's protocol describes the characteristics that all patients in the study must have. Called eligibility criteria, these guidelines differ from study to study, depending on the research purpose. They may include age, gender, the type and **stage** of cancer, and whether cancer patients who have had prior cancer treatment or who have other health problems can take part.

Using eligibility criteria is an important principle of medical research that helps produce reliable results. During a study, they help protect patient safety, so that people who are likely to be harmed by study drugs or other treatments are not exposed to the risk. After results are in, they also help doctors know which patient groups will benefit if the new treatment being studied is proven to work. For instance, a new treatment may work for one type of cancer but not for another, or it may be more effective for men than women.

3. Cancer clinical trials include research at three different phases.

Each phase answers different questions about the new treatment.

- **Phase I** trials are the first step in testing a new treatment in humans. In these studies, researchers look for the best way to give a new treatment (e.g., by mouth, IV drip, or injection? how many times a day?). They also try to find out if and how the treatment can be given safely (e.g., best dose?); and they watch for any harmful **side effects.** Because less is known about the possible risks and benefits in Phase I, these studies usually include only a limited number of patients who would not be helped by other known treatments.

- **Phase II** trials focus on learning whether the new treatment has an anticancer effect (e.g., Does it shrink a tumor? improve blood test results?). As in Phase I, only a small number of people take part because of the risks and unknowns involved.

- **Phase III** trials compare the results of people taking the new treatment with results of people taking standard treatment (e.g., Which group has better survival rates? fewer side effects?). In most cases, studies move into Phase III testing

only after a treatment shows promise in Phases I and II. Phase III trials may include hundreds of people around the country.

4. In Phase III trials, people are assigned at random to receive either the new treatment or standard treatment.

Researchers assign patients by chance either to a group taking the new treatment (called the **treatment group**) or to a group taking standard treatment (called the **control group**). This method, called **randomization,** helps avoid **bias:** having the study's results affected by human choices or other factors not related to the treatments being tested.

In some studies, researchers do not tell the patient whether he or she is in the treatment or control group (called a **single blind study**). This approach is another way to avoid bias, because when people know what drug they are taking, it might change the way they react. For instance, patients who knew they were taking the new treatment might expect it to work better and report hopeful signs because they want to believe they are getting well. This could bias the study by making results look better than they really were.

Why Do Phase III Clinical Trials Compare Treatment Groups?

Comparing similar groups of people taking different treatments for the same type of cancer is another way to make sure that study results are real and caused by the treatment rather than by chance or other factors. Comparing treatments with each other often shows clearly which one is more effective or has fewer side effects.

Another reason Phase III trials compare the new treatment with standard treatment is so that *no one in a study is left without any treatment when standard treatment is available,* which would be unethical. When no standard treatment exists for a cancer, some studies compare a new treatment with a **placebo** (a look-alike pill that contains no active drug). However, you will be told if this is a possibility before you decide whether to take part in a study.

Your Doctor Can Tell You More

If you have any questions about how clinical trials work, ask your doctor, nurse, or other health professional. It may be helpful to bring this booklet and discuss points you want to understand better.

Should I Take Part in a Clinical Trial?

This is a question only you, those close to you, and your health professionals can answer together. Learning you have cancer and deciding what to do about it is often overwhelming. This section has information you can use in thinking about your choices and making your decision.

Clinical Trials: Weighing the Pros and Cons

While a clinical trial is a good choice for some people, this treatment option has possible benefits and drawbacks. Here are some factors to consider. You may want to discuss them with your doctor and the people close to you.

Possible Benefits

- Clinical trials offer high-quality cancer care. If you are in a study and do not receive the new treatment being tested, you will receive the best standard treatment. This may be as good as, or better than, the new approach.
- If a new treatment approach is proven to work and you are taking it, you may be among the first to benefit.
- By looking at the pros and cons of clinical trials and your other treatment choices, you are taking an active role in a decision that affects your life.
- You have the chance to help others and improve cancer treatment.

Possible Drawbacks

- New treatments under study are not always better than, or even as good as, standard care. They may have side effects that doctors do not expect or that are worse than those of standard treatment.
- Even if a new treatment has benefits, it may not work for you. Even standard treatments, proven effective for many people, do not help everyone.
- If you receive standard treatment instead of the new treatment being tested, it may not be as effective as the new approach.
- Health insurance and managed care providers do not always cover all patient care costs in a study. What they cover varies by plan and by study. To find out in advance what costs are likely to be paid in your case, talk to a doctor, nurse or social worker from the study.

Your Rights, Your Protections

Before and during a cancer treatment study, you have a number of rights. Knowing these can help protect you from harm.

- Taking part in a treatment study is up to you. It may be only one of your treatment choices. Talk with your doctor. Together, you can make the best choice for you.
- If you do enter a study, doctors and nurses will follow your response to treatment carefully throughout the research.
- If researchers learn that a treatment harms you, you will be taken off the study right away. You may then receive other treatment from your own doctor.
- You have the right to leave a study at any time.

One of your key rights is the right to **informed consent.** Informed consent means that you must be given all the facts about a study before you decide whether to take part. This includes details about the treatments and tests you may receive and the possible benefits and risks they may have. The doctor or nurse will give you an informed consent form that goes over key facts. If you agree to take part in the study, you will be asked to sign this informed consent form.

The informed consent process continues throughout the study. For instance, you will be told of any new findings regarding your clinical trial, such as new risks. You may be asked to sign a new consent form if you want to stay in the study.

Signing a consent form does not mean you must stay in the study. In fact, you can leave at any time. If you choose to leave the study, you will have the chance to discuss other treatments and care with your own doctor or a doctor from the study.

Questions You Should Ask

Finding answers and making choices may be hard for people with cancer and those who care about them. It is important to discuss your treatment choices with your doctor, a cancer specialist (an **oncologist**) to whom your doctor may refer you, and the staff of any clinical trial you consider entering.

Ask questions about the information you receive during the informed consent process and about any other issues that concern you. Getting answers can help you work better with the doctor. You may want to take a friend or relative along when you talk to the doctor. It also may help to write down your questions and the answers you receive, or bring a tape recorder to record what is said. No question about your care is foolish. It is very important to understand your choices.

Here are some questions you may want to ask about:

The Study

- What is the purpose of the study? In what phase is this study?
- Why do researchers believe the new treatment being tested may be effective? Has it been tested before?
- Who sponsors the study, and who has reviewed and approved it?
- How are the study data and patient safety being checked?
- When and where will study results and information go?

Possible Risks and Benefits

- What are the possible short- and long-term risks, side effects, and benefits to me?

- Are there standard treatments for my type of cancer?
- How do the possible risks, side effects, and benefits in the study compare with standard treatment?

Your Care

- What kinds of treatments, medical tests, or procedures will I have during the study? Will they be painful? How do they compare with what I would receive outside the study?
- How often and for how long will I receive the treatment, and how long will I need to remain in the study? Will there be follow-up after the study?
- Where will my treatment take place? Will I have to be in the hospital? If so, how often and for how long?
- How will I know if the treatment is working?
- Will I be able to see my own doctor? Who will be in charge of my care?

Personal Issues

- How could the study affect my daily life?
- Can you put me in touch with other people who are in this study?
- What support is there for me and my family in the community?

Cost Issues

- Will I have to pay for any treatment, tests, or other charges?
- What is my health insurance likely to cover?
- Who can help answer any questions from my insurance company or managed care plan?

Other Questions

(Use this space to write down other questions you have.)

Others Can Help

As you make your treatment decisions, remember that you are not alone. Doctors, nurses, social workers, other people with cancer, clergy, family, and those who care about you can help and support you. The resources in the next section also can provide more information and links to other contacts in your community.

National Cancer Institute Information Resources

You may want more information for yourself, your family, and your doctor. The following National Cancer Institute (NCI) services are available to help you.

Telephone...

Cancer Information Service (CIS)
Provides accurate, up-to-date information on cancer to patients and their families, health professionals, and the general public. Information specialists translate the latest scientific information into understandable language and respond in English, Spanish, or on TTY equipment.

Toll-free: **1-800-4-CANCER** (1-800-422-6237)
TTY: 1-800-332-8615

Internet... these web sites may be useful:

http://www.nci.nih.gov
NCI's primary web site; contains information about the Institute and its programs.

http://cancertrials.nci.nih.gov
Cancer Trials; NCI's comprehensive clinical trials information center for patients, health professionals, and the public. Includes information on understanding trials, deciding whether to participate in trials, finding specific trials, plus research news and other resources.

http://cancernet.nci.nih.gov
CancerNet™; contains material for health professionals, patients, and the public, including information from PDQ® about cancer treatment, screening, prevention, supportive

care, and clinical trials, and CANCERLIT®, a bibliographic database.

http://rex.nci.nih.gov
Includes news, upcoming events, educational materials, and publications for patients, the public, and the mass media.

http://chid.nih.gov/ncichid/
Cancer Patient Education Database; provides information on cancer patient education resources for health educators and other health professionals.

E-mail...

CancerMail
Includes NCI information about cancer treatment, screening, prevention, and supportive care. To obtain a contents list, send e-mail to **cancermail@icicc.nci.nih.gov** with the word "help" in the body of the message.

Fax...

CancerFax®
Includes NCI information about cancer treatment, screening, prevention, and supportive care. To obtain a contents list, dial **301-402-5874** from a fax machine hand set and follow the recorded instructions.

Glossary

This glossary contains a list of words used in the booklet and their definitions. It also explains some other terms related to treatment studies that you may hear from your doctor or nurse.

Bias: Human choices or any other factors beside the treatments being tested that affect a study's results. Clinical trials use many methods to avoid bias, because biased results may not be correct.

Clinical trials: Research studies that involve people. Each study tries to answer scientific questions and to find better ways to prevent or treat cancer.

Control group: In a clinical trial, the group of people that receives standard treatment for their cancer. *(See Treatment group.)*

Informed consent: The process in which a person learns key facts about a clinical trial or research study and then agrees voluntarily to take part or decides against it. This process includes signing a form that describes the benefits and risks that may occur if the person decides to take part.

Institutional Review Board (IRB): Groups of scientists, doctors, clergy, and consumers at each health care facility at which a clinical trial takes place. Designed to protect patients who take part in studies, IRBs review and must approve the protocols for all clinical trials funded by the Federal Government. They check to see that the study is well-designed, does not involve undue risks, and includes safeguards for patients.

Investigator: A researcher in a treatment study.

Oncologist: A doctor who specializes in treating cancer.

Placebo: A tablet, capsule, or injection that looks like the drug or other substance being tested but contains no drug.

Protocol: An action plan for a clinical trial. The plan states what will be done in the study and why. It outlines how many people will take part in the study, what types of patients may take part, what tests they will receive and how often, and the treatment plan.

Randomization: A method used to prevent bias in research. People are assigned by chance to either the treatment or control group.

Remission: When the signs and symptoms of cancer go away, the disease is said to be "in remission." A remission can be temporary or permanent.

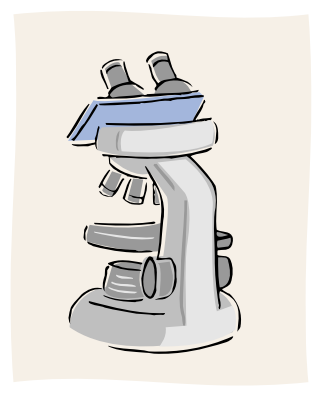
Side effects: Problems that occur when treatment affects healthy cells. Common side effects of standard cancer treatments are fatigue, nausea, vomiting, decreased blood cell counts, hair loss, and mouth sores. New treatments being tested may have these or other unknown side effects.

Single blind study: A method used to prevent bias in treatment studies. In a single blind study, the patient is not told whether he/she is taking the standard treatment or the new treatment being tested. Only the doctors know.

Stage: The extent of a cancer and whether the disease has spread from the original site to other parts of the body. Numbers with or without letters are used to define cancer stages (e.g., Stage IIb).

Standard treatment: The best treatment currently known for a cancer, based on results of past research.

Treatment group: The group that receives the new treatment being tested during a study. *(See Control group.)*



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