National Cancer Institute

Taking Part in Cancer Treatment Research Studies











U.S. DEPARTMENT OF HEALTH AND HUMAN SERVICES
National Institutes of Health

Taking Part in Cancer Treatment Research Studies

If you have cancer, you may want to think about taking part in a clinical trial. Clinical trials are a treatment option for many people with cancer. This book explains cancer treatment clinical trials and gives you some things to think about when deciding whether to take part.

This booklet is for people with cancer, their family, and friends.

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What are clinical trials?

Clinical trials are research studies that involve people. They are the final step in a long process that begins with research in a lab and animal testing. Many treatments used today are the result of past clinical trials.

In cancer, clinical trials are designed to answer questions about new ways to:

- Treat cancer
- Find and diagnose cancer
- Prevent cancer
- Manage symptoms of cancer or side effects from its treatment

This booklet focuses on cancer treatment studies. These studies are designed to answer questions about new treatments or ways of using existing treatments better. These trials test many types of treatments, such as new:

- Drugs or vaccines
- Ways to do surgery or give radiation therapy
- Combinations of treatments

Many treatments used today are the results of past clinical trials.

Clinical trials take place in phases

For a treatment to become standard, it must first go through 3 or 4 clinical trial phases. The early phases make sure the treatment is safe. Later phases show if it works better than the standard treatment. You do not have to take part in all phases.

Phase I

Purpose:

- To find a safe dose
- To decide how the new treatment should be given
- To see how the new treatment affects the human body

Number of people taking part: 15–30

Phase II

Purpose:

- To determine if the new treatment has an effect on a certain cancer
- To see how the new treatment affects the human body

Number of people taking part: Less than 100

Phase III

Purpose:

- To compare the new treatment (or new use of a treatment) with the current standard treatment

Number of people taking part: From 100 to several thousand

Phase IV

Purpose:

- To further assess the long-term safety and effectiveness of a new treatment

Number of people taking part: Several hundred to several thousand

Clinical trials follow strict guidelines

The guidelines that clinical trials follow clearly state who will be able to join the study and the treatment plan. Every trial has a person in charge, usually a doctor, who is called the principal investigator. The principal investigator prepares a plan for the study, called a protocol, which is like a recipe for conducting a clinical trial.

The protocol explains what the trial will do, how the study will be carried out, and why each part of the study is necessary. It includes information about:

- The reason for doing the study
- Who can join the study
- How many people are needed for the study
- Any drugs they will take, the dose, and how often
- What medical tests they will have and how often
- What information will be gathered about them

Who can join a clinical trial?

Based on the questions the research is trying to answer, each clinical trial protocol clearly states who can or cannot join the trial.

Common criteria for entering a trial include:

- Having a certain type or stage of cancer
- Having received a certain kind of therapy in the past
- Being in a certain age group

Criteria such as these help ensure that people in the trial are as alike as possible. This way doctors can be sure that the results are due to the treatment being studied and not other factors.

These criteria also help ensure:

- **Safety**

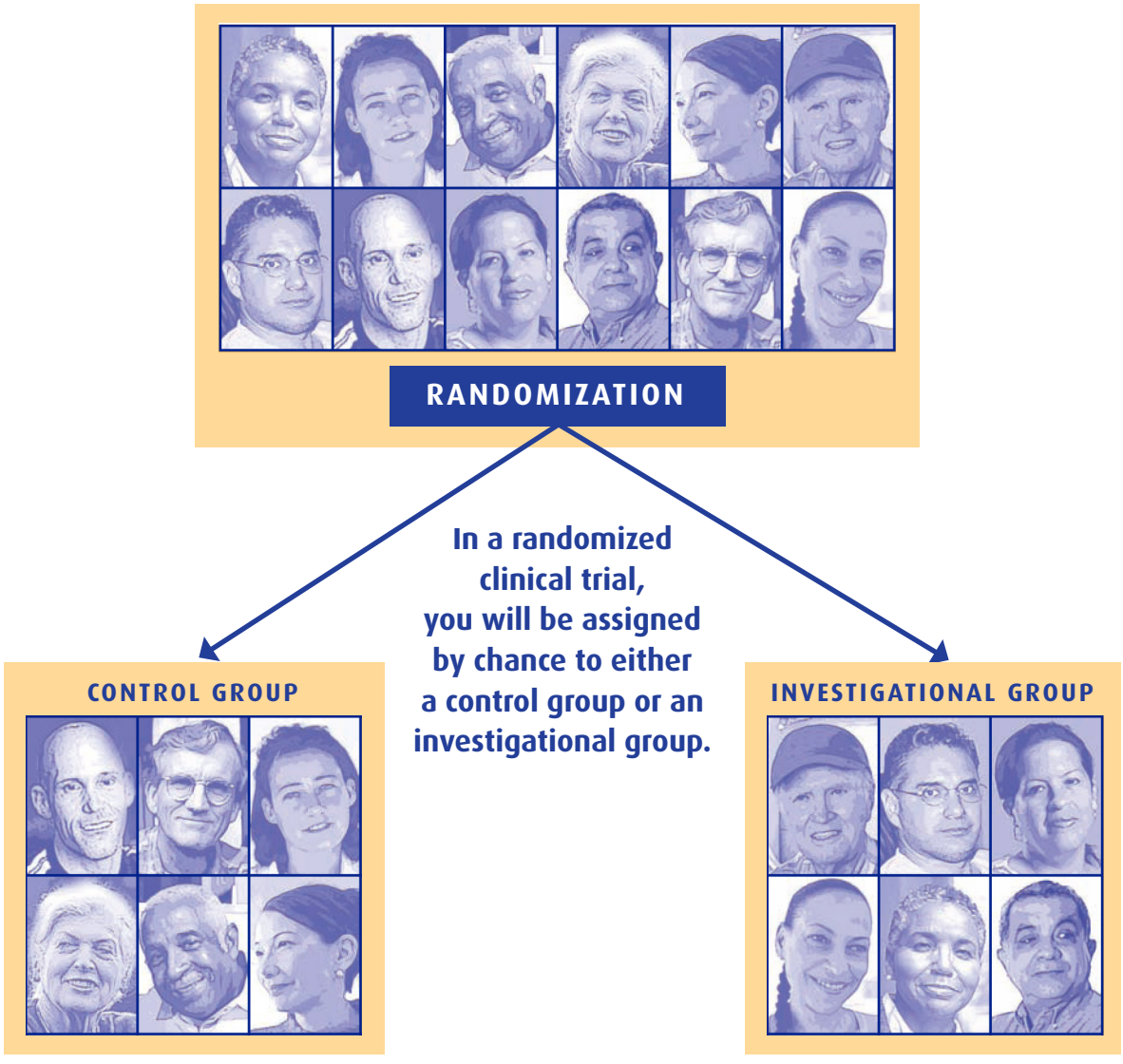
  Some people have health problems besides cancer that could be made worse by the treatments in a study. If you are interested in joining a trial, you will receive medical tests to be sure that you are not put at increased risk.

Accurate and meaningful study results

You may not be able to join some clinical trials if you already have had another kind of treatment for your cancer. Otherwise, doctors could not be sure whether your results were due to the treatment being studied or the earlier treatment.

Randomization

Randomization is a process used in some clinical trials to prevent bias. Bias occurs when a trial's results are affected by human choices or other factors not related to the treatments being tested. Randomization helps ensure that unknown factors do not affect trial results.



Randomization is used in all phase III and some phase II trials. These trials are called randomized clinical trials.

If you participate in such a trial, you will be assigned by chance to either an investigational group or a control group. Your assignment will be determined with a computer program or table of random numbers.

- If you are assigned to the control group, you will get the most widely accepted treatment (standard treatment) for your cancer.
- If you are assigned to the investigational group, you will get the new treatment being tested.

Comparing these groups to each other often clearly shows which treatment is more effective or has fewer side effects. If you are thinking about joining a randomized clinical trial, you need to understand that you have an equal chance to be assigned to either one of the groups. Neither you nor your doctor chooses which group you will be in.

Will I get a placebo?

A placebo is designed to look like the medicine being tested, but it is not active. **Placebos are almost never used in cancer treatment trials.** In some cases, a study may compare standard treatment plus a new treatment, to standard treatment plus a placebo. You will be told if the study uses a placebo.

Patient protection

Federal rules help ensure that clinical trials are run in an ethical manner. Your rights and safety are protected through:

- Informed consent
- Careful review and approval of the clinical trial protocol by two review panels. These panels include:
  - A scientific review panel
  - An institutional review board (IRB)
- Ongoing monitoring provided during the trial by:
  - The IRB
  - Data and Safety Monitoring Boards (DSMBs)
  - Your research team

Informed Consent

Informed consent is a process through which you learn the purpose, risks, and benefits of a clinical trial before deciding whether to join. It is a critical part of ensuring patient safety in research. During the informed consent process you learn important information about a clinical trial. This information can help you decide whether to join.

During the informed consent process, you learn important information about the clinical trial that can help you decide whether to take part.

The research team, which is made up of doctors and nurses, first explains the trial to you. The team explains the trial's:

- Purpose
- Tests and procedures
- Treatment
- Risks and benefits

They will also discuss your rights, including your right to:

- Make a decision about participating
- Leave the study at any time

If you decide to leave the study, your doctor will discuss other treatment options with you.

Before agreeing to take part in a trial, you have the right to:

- Learn about all your treatment options
- Learn all that is involved in the trial—including all details about treatment, tests, and possible risks and benefits
- Discuss the trial with the principal investigator and other members of the research team
- Both hear and read the information in language you can understand

After discussing all aspects of the study with you, the team gives you an informed consent form to read. The form includes written details about the information that was discussed and also describes the privacy of your records. If you agree to take part in the study, you sign the form. But even after you sign the consent form, you can leave the study at any time.

Most clinical trials have to go through different types of review that are designed to protect all people who take part. These reviews are conducted by scientific review panels, Institutional Review Boards (IRBs), and Data and Safety Monitoring Boards (DSMBs).

Scientific Review Panels

This panel is made up of experts who review a clinical trial protocol before it starts accepting patients to make sure it is based on sound science. All clinical trials that are funded by the Government must go through this review. Many other clinical trial sponsors, such as drug companies, also seek expert advice on the scientific merit of their trial protocols.

Institutional Review Boards

This board also reviews a clinical trial protocol before it starts accepting patients. The board members make sure the risks involved in the trial are reasonable when compared to the possible benefits. They also closely watch the ongoing progress of the trial from beginning to end.

Federal rules require that each IRB be made up of at least 5 people. One member must be from outside the institution running the trial. IRBs are usually made up of a mix of medical specialists and members of the community.

Many include members from diverse careers and backgrounds. In most cases IRBs are located where the trial is to take place. Many institutions that carry out clinical trials have their own IRBs.

Data and Safety Monitoring Boards (DSMBs)

For phase III trials, DSMBs monitor the trial to help ensure your safety. They may also be appropriate and necessary for certain phase I and II clinical trials. A DSMB is an independent committee made up of statisticians, physicians, and other experts.

The Board must:

- Ensure that any risks that come from being in the study are reduced as much as possible
- Ensure that the data are sound
- Stop a trial if safety concerns come up or as soon as its objectives have been met

Deciding to take part in clinical trials

Whenever you need treatment for your cancer, clinical trials may be an option for you. Choosing to join a clinical trial is something only you, those close to you, and your doctors and nurses can decide together. This section has information you can use when thinking about your treatment choices and making your decision.

Weighing the Pros and Cons

As with any treatment option, a clinical trial has possible benefits as well as drawbacks. You may want to discuss the following issues with your doctor and the people close to you.

Possible Benefits

- Clinical trials offer high-quality cancer care. If you are in a randomized study and do not receive the new treatment being tested, you will receive the best known standard treatment. This may be as good as, or better than, the new approach.
- If a new treatment is proven to work and you are receiving it, you may be among the first to benefit.
- By looking at all your treatment choices, including clinical trials, 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.
- If you receive standard care instead of the new treatment being tested, it may not be as effective as the new approach.
- New treatments 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.
- Health insurance and managed care providers may not 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 covered, check with your insurance company and the billing staff at the hospital or doctor's office.

Questions to ask

If you are thinking about taking part in a clinical trial, here are some questions that can help you decide.

About the trial

- Why is this trial being done?
- Why do the doctors who designed the trial believe that the treatment being studied may be better than the standard treatment? Why may it not be better?
- How long will I be in the trial?
- What kinds of tests and treatments are involved?
- What are the possible side effects or risks of the new treatment?
- What are the possible benefits?
- How will we know if the treatment is working?

Costs

- Will I have to pay for any of the treatments or tests?
- What costs will my health insurance cover?

Daily life

- How could the trial affect my daily life?
- How often will I have to come to the hospital or clinic?
- Will I have to travel long distances to take part?

Comparing choices

- What are my other treatment choices, including standard treatments?
- How does the treatment I would receive in this trial compare with the other treatment choices?

How to find clinical trials

The National Cancer Institute, drug companies, medical institutions, and other organizations sponsor clinical trials. Clinical trials take place in many settings, such as cancer centers, large medical centers, small hospitals, and doctors' offices.

The National Cancer Institute maintains a list of thousands of cancer clinical trials. Contact us to see which ones you might be eligible for.

To reach the **National Cancer Institute**:

Call: 1-800-4-CANCER (1-800-422-6237)

Visit: www.cancer.gov

Chat: www.cancer.gov/livehelp

E-mail: cancergovstaff@mail.nih.gov

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