LEUKEMIA & LYMPHOMA SOCIETY OF CANADA®

Understanding CLINICAL TRIALS

WHAT YOU NEED TO KNOW

You or your loved one has been diagnosed with a type of blood cancer. You may be offered treatment through a clinical trial. What does it mean and how does it work?

This fact sheet will help you:

- Get an overview of clinical trials
- Understand clinical trials and how they are planned
- Evaluate the benefits and risks of participating
- Address your safety concerns
- Determine what questions to ask

What is a clinical trial?

A **clinical trial** is a research study that aims to evaluate a medical intervention in human volunteers or patients. Its goal is to improve cancer care and treatment by testing new drugs and therapies to see how safe and helpful they are. Trials are always taking place, as doctors and researchers are always looking for new and better treatments.

About clinical trials

Researchers design clinical trials to study new ways to:

- Find and diagnose cancer
- Treat cancer using:
  - A new drug
  - A new drug combination
  - A new way to deliver a drug
  - A therapy that is approved for a different disease
- Manage cancer symptoms
- Reduce side effects and manage long-term side effects of treatment
- Prevent cancer from returning
- Improve people's response rates to treatment
- Improve overall survival rates
- Better understand each disease

Many clinical trials for cancer search for a cure by developing safer and more effective treatments to destroy cancer cells and keep them from coming back.

Other clinical trials look for new ways to refine existing treatments and improve the quality of life for people living with cancer.

Before the clinical trial

When researchers want to evaluate a new drug or a new approach to therapy, they conduct a **pre-clinical trial,** or early research. The pre-clinical trial involves testing in a laboratory. It provides initial information on safety, how well the drug works, and the dose to be used in clinical trials. The treatment goes through approvals before it can be tested in humans in a clinical trial.

The clinical trial

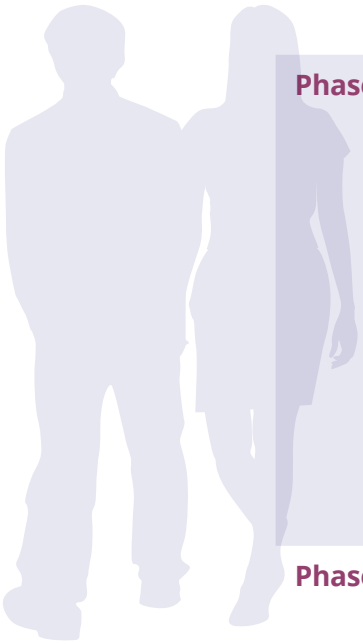
For a drug to become a standard treatment, it must be tested and go through a series of steps called phases. Development of a cancer drug usually has four phases of clinical trials.

- The early phases are to determine whether the treatment is safe and effective.
- The later phases are to evaluate if the new therapy works better than the standard treatment.

Standard treatment (also known as standard therapy or best practice) is treatment that medical experts commonly use and accept as being appropriate for your disease.

Each phase must be completed successfully before the drug can move to the next phase. The four phases of a clinical development or testing of a new drug include:

Phase	Description
Phase 1	• Purpose: To test the safety, side effects, and dosage. To determine the best way to give the treatment. • Group size: 20 or more people • May be the first time the drug is tested in humans
Phase 2	• Purpose: To determine the safety and effectiveness for a specific cancer. To identify side effects. • Group size: Often fewer than 100 and up to 300 people
Phase 3	• Purpose: To confirm the effectiveness, side effects, and safety in a larger group of patients and to compare it to other treatments. • Group size: Between 300 and several thousand people • People are randomly put into groups to study different approaches to treatment. - **Single-blind study**: You don't know if you're getting the study treatment or the standard treatment. - **Double-blind study:** Neither you nor your healthcare team know if you're getting the study treatment or the standard treatment. - **Open-label trial:** Both you and your healthcare team know which treatment you're getting.
Phase 4	• Purpose: To gather more information on safety and effectiveness after Health Canada approves the drug and it is on the market. • Group size: Several thousand people.



Should you participate in a clinical trial?

Taking part in a clinical trial may be the best treatment for some people with a blood cancer. There are trials happening at every stage of treatment and for people in remission. Whether you should participate is a very personal decision.

Take an active role in your care by talking to your healthcare team about:

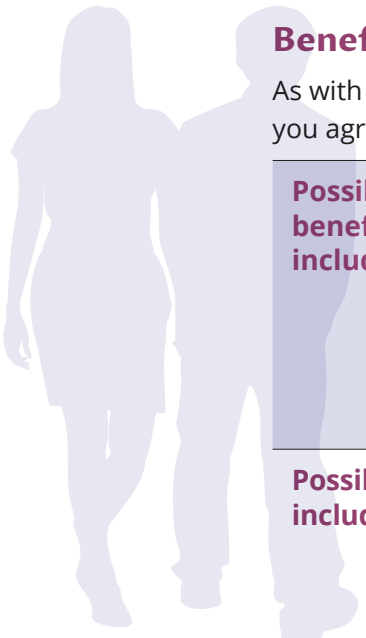
- Your type of cancer
- Your overall health
- The types of clinical trials available

Clinical trials of new drugs and treatments are essential to making progress with cancer. Today, there are thousands of clinical trials for leukemia, lymphoma, myeloma, myelodysplastic syndromes, myeloproliferative neoplasms, and other blood cancers. People are living longer because of successful cancer treatments that are the result of past clinical trials.

14% vs 90%

The survival rate for children with acute lymphoblastic leukemia (ALL) is 90% today compared to 14% in 1966.

Is a clinical trial for you?	You don't need to wait to find out your standard treatment is not working before you consider a clinical trial. You might be eligible to participate in a trial designed to test new treatments if you are newly diagnosed, have a limited amount of cancer, or are in remission. It's important to ask your doctor about whether a clinical trial is an option for you. Eligibility criteria may depend on: • Your type of cancer • The stage of your cancer • Your age and gender • Your overall health • Other treatments you are taking • The results you've had with standard treatment
What if you're not eligible	If you are not eligible to participate in a clinical trial, there may be other ways to access treatments that are not yet approved. Expanded access, also known as compassionate use, may give you access to drugs being investigated outside of a clinical trial when there is no alternative treatment for you. Talk to your healthcare team to see if this is an option.



Benefits and risks

As with any treatment option, a clinical trial can have benefits and risks. Talk to your doctor before you agree to participate.

Possible benefits include	• Early access to a promising new treatment • High-quality care from the trial's healthcare team as they monitor your cancer and side effects • Access to doctors with extensive experience treating your type of cancer • Helping to advance cancer research
Possible risks include	• Unknown side effects • The new drug or treatment does not work for you • More frequent visits to the doctor and more tests and exams • Travel costs to get to the clinical trial site may not be covered • May affect your family and work responsibilities, depending on side effects, time commitment, and travel needs

Are clinical trials safe?

The safety of people participating in a clinical trial is the most important thing. All trials follow strict scientific and ethical principles.

Every trial requires steps to protect patients:

Requirement	Description
Principal investigator	The person in charge of the study, usually a doctor. Their role is to develop a study plan or protocol.
Study protocol	The aim of a clinical trial is to provide answers to important research questions. The study protocol describes how this will be done in a systematic way to ensure a scientific and ethical approach to evaluating the risks and benefits of a new drug. Every doctor at every treatment centre taking part in the trial must follow the study protocol.
Eligibility criteria	These guidelines cover who is eligible to participate. Not everyone who is interested is accepted. Eligibility criteria include type and stage of cancer, age, and health status. These help to ensure safety and accurate results.
Informed consent	This should clearly explain the potential benefits and risks to participants. A research team member will explain the details of the study to you. Learn all you can about the clinical trial so you can decide whether to participate. You can review the details at home and with your doctor. Ask questions. Find out what to expect with tests, costs, and the option of saying no. You will be asked to sign an informed consent form stating that you fully understand what's involved in the study. You are free to leave the study at any time.
Informed consent for children	The safety of children is the greatest priority in research studies. Children have special protection. The risks must be balanced with the need to develop safe and effective treatments for children. In most cases, both the child's parents/guardians must give legal consent for the child to participate. Once children are of legal age, they can sign their own consent forms. It's a good idea to discuss the decision to participate with your child. This will help them learn about the clinical trial. They can also ask questions. Children ages 7 and older will generally be asked for their "assent," meaning they agree to take part in the trial. Parents/guardians can take their child out of a research study at any time, for any reason.

Scientific review	Procedures are in place to make sure that clinical trials are done safely and ethically. These safeguards are proposed by the sponsoring organization and reviewed by the principal investigator, the scientific team at Health Canada, the Institutional Review Board, and the Data Safety Monitoring Board. Each reviews the proposed clinical trial protocol to confirm that the: • information from pre-clinical and clinical tests or trials conducted so far can ensure the safe and ethical conduct of the clinical trial; • number of patients, types of medical tests, and proposed dosage are appropriate for the stage of clinical trial; and • plan described in the protocol will ensure a study that is done fairly and safely, and will meet scientific and clinical standards.

Common questions about clinical trials

Will I get only a placebo?	No. A placebo is an inactive pill, liquid, or powder that looks like a drug but has no therapeutic effect on your disease. In clinical trials for cancer, placebos are not used unless they are given along with an active drug.
Will I be in danger if I do a clinical trial?	No. Clinical trials are carefully designed to put the health and safety of people with cancer first. You can ask questions and will provide informed consent before you begin.
Can I pick the treatment group?	No. Treatment groups are chosen randomly. You have an equal chance of being put in any of the groups. You can expect to receive excellent care whether you are receiving a new treatment or a standard treatment.

How to find a clinical trial

Talk to your healthcare team about finding a clinical trial that might work for you. If you find a clinical trial that you want to know more about, your healthcare team can help by reviewing the eligibility criteria with you. They know your type of cancer, the stage, treatments, and responses to your treatment. You can also call us to be connected with a clinical trial nurse navigator.

Questions to ask your clinical trial team

Ask questions so you understand more about the trial, the risks, and the benefits. Here are some questions you may want to ask:

About the study	• What kind of treatment is involved? • Why do researchers think it may be effective? • Has the treatment been used with other types of cancers? • What were the results in earlier studies and stages of this treatment? • Where can I find more information?

About your health	• What are the risks and benefits compared to the standard treatment? • What are the possible short-term and long-term side effects of the standard treatment and the new treatment? • How long does the treatment last? • How will I know if the treatment is working? • What happens if my health gets worse during the trial? • Can I talk to other people in the study? • Do I need to keep a log or fill out forms about my health during the trial? • How is my privacy protected?
About the cost	• Where is the trial taking place? • Will I have to pay for any costs related to the trial, such as travel or childcare? • Will my insurance company cover any of these costs?
About follow-up care	• If the treatment is effective, can I continue the treatment after the study ends? • Will the study team continue to check me after the treatment is over? • Will I have to travel to the site for follow-up care or can it be done locally? • Will I get the general results of the trial?

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