

Understanding Clinical Trials for Blood Cancers



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Support for this publication provided by



A six-word narrative about living with blood cancer from patients in our LLS Community

Stay strong and keep moving forward. Find the positive in every day. Be your own best patient advocate. Changed my life for the better. Accept, learn and focus on present. Learning to live a different life. Sudden and life changing—be positive. Waiting, worrying, anxiousness/happy I'm alive! Embrace a new normal each day. 5 years, 41 infusions, constant fatigue. Patience, positive attitude, hope and faith. Test to test, I will survive! Treatment, fatigue, treatment, fatigue and survival. Love life, live better every day. I don't look back only forward. So far, so good, live life. Meditation, mindfulness, wellness, faith, nutrition and optimism. Finding the joy while living with uncertainty. Watch, wait, treat, regroup, rest, re-energize. Blessed to be doing so well! Eye opening needed learning and healing. Feel great: uncertain travel plans annoying. Renewed faith, meditation, diet, mindfulness, gratitude. Watchful waiting can be watchful worrying. Scary, expensive, grateful, blessings, hope, faith. Thank god for stem cell transplants! Do not know what to expect. Extraordinarily grateful, I love my life. Diagnosed; frightened; tested; treating; waiting; hoping. I'm more generous, impatient less often. Embrace your treatment day after day. Live today, accept tomorrow, forget yesterday. Strength you never realized you had. Challenging to our hearts and minds. Life is what we make it. Live life in a beautiful way.



Discover what thousands already have at
www.LLS.org/Community

Join our online social network for people who are living with or supporting someone who has a blood cancer. Members will find

- Thousands of patients and caregivers sharing experiences and information, with support from knowledgeable staff
- Accurate and cutting-edge disease updates
- The opportunity to participate in surveys that will help improve care.

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The nurse navigators at The Leukemia & Lymphoma Society's Clinical Trial Support Center.

Introduction

If you or someone you love has cancer, you may have many questions about available treatment options. Treatment options include standard medical care or treatment in a clinical trial.

Standard medical care (also called “standard therapy” or “best practice”) is treatment that is widely used and accepted by medical experts as appropriate treatment for a certain type of disease.

A clinical trial for new cancer drugs and treatments is a carefully controlled research study that aims to improve the care and treatment of cancer patients. In the United States, the Food and Drug Administration (FDA) requires all new drugs and other treatments to be tested in clinical trials before they are made available to the public. At any given time, there are thousands of cancer clinical trials available as doctors and researchers are always seeking new and better treatments for patients.

Many cancer clinical trials are searching for a cure. This means devising safer, more effective treatments that destroy cancer cells and keeps them from coming back. Other clinical trials look for new ways to improve existing treatments and to improve the quality of life for patients. Researchers design cancer clinical trials to study new ways to

- Treat cancer using
 - A new drug
 - An approved therapy for a different diagnosis
 - A new drug combination
 - A new way of delivering a drug (pill, intravenously (IV), etc).
- Manage cancer symptoms and alleviate the side effects of treatment
- Find and diagnose cancer
- Prevent cancer from returning
- Manage long-term side effects.

Taking part in a clinical trial may be the best treatment choice for some blood cancer patients. There are trials for patients at every stage of treatment as well as for those whose disease is in remission. All of today’s standard treatments for cancer are based on the findings from previous clinical trials.

This booklet will help you to know more about how new treatments are developed, what happens during a clinical trial, how clinical trials help advance blood cancer treatment, how to evaluate the benefits and risks of a clinical trial for yourself, what questions to ask when deciding if a clinical trial is right for you and how The Leukemia & Lymphoma Society (LLS) can help.

Talk With Your Doctor

Treatment decisions are very personal. Your type of cancer, overall condition and priorities are all important considerations that should be addressed when you discuss treatment options with your doctor. Be aware that a clinical trial may be the best treatment option for you at some point during your cancer care. It is important for you to have an opportunity to discuss all of your treatment options, including clinical trials, with your doctor.

When you discuss treatment options with your doctor, it may be helpful to have

- A list of questions to ask (see *Question Guides* on page 30 and/or you can download other question guides at www.LLS.org/whattoask)
- A family member, friend or another advocate go to your doctor's visit with you—both for support and to take notes.

If your doctor does not suggest a clinical trial as a potential treatment for you, ask whether this might be an option. By looking at all of your treatment options, you are taking an active role in your care. Some doctors may either not be aware of an appropriate clinical trial or may not recommend one. Getting another opinion about your treatment from a doctor who specializes in blood cancer (hematologist-oncologist) may help you to find additional clinical trial options.

Today, there are thousands of clinical trials for leukemia, lymphoma, myeloma, myelodysplastic syndromes, myeloproliferative neoplasms and other blood cancers. These trials are designed to improve treatment response rates and overall survival, reduce disease and treatment side effects, and provide a better understanding of each disease. By participating in clinical trials, doctors and patients are learning about these diseases and building new bridges to improved outcomes and quality of life.

The Importance of Patient Participation in Clinical Trials

Clinical trials of new drugs and treatments are essential in making progress against cancer. Today, people are living longer because of successful cancer treatments that were the outcome of past clinical trials. The progress made in treating children with acute lymphoblastic leukemia (ALL), the most common form of childhood cancer, is a striking example of successful cancer treatment made possible from clinical trials. Historically, enrollment of children in cancer clinical trials has been much higher than enrollment of adults. As a result, there have been great improvements in survival rates of children, particularly children diagnosed with ALL. In 1966, the survival rate for these children was only 14 percent. Today, as a result of clinical trials and successful treatments, more than 90 percent of children survive this form of leukemia. Patients taking part in clinical trials today help to improve cancer care for future patients.

Patient diversity in clinical trials is also critical to public health. Researchers now know that a patient's response to a treatment can vary due to a number of factors including age, gender, genetics, ethnic origin and weight. These differences can play a role in the safety and effectiveness of a medication. Differences among people can lead to different responses to the same medication. For example, one group of people can have more side effects to a treatment compared to the side effects experienced by another group of people who are receiving the same treatment. Therefore, it is essential to test treatments in a diverse population. When clinical trials include a diverse group of patients, the results of the study may be much more relevant to the general population. Joining a clinical trial may not only benefit you, it may benefit your community as well.

Who Can Participate in a Cancer Clinical Trial? Your doctor may talk to you about treatment in a clinical trial. However, many patients seen by a hematologist-oncologist say that their specialist never informed them about the possibility of participating in a trial. Blood cancer patients (or their advocates) may need to ask their doctors about clinical trials.

Some patients may think they should wait until standard treatment fails before they consider a clinical trial. However, clinical trials are not only for patients with the most advanced disease. A trial can be designed to test new treatment(s) in newly diagnosed patients, patients with very limited disease, or patients whose disease is in remission and/or who are on maintenance therapy.

Clinical trials are appropriate treatment options for different types of patients, depending on the purpose and phase of the trial. Patient eligibility criteria may include, but is not limited to,

- Disease type
- Stage of disease
- Patient age/gender
- Other treatments used by the patient
- The presence of any other illnesses or conditions

Some trials might require that you try standard treatment before you can participate in those trials; other trials may be enrolling patients who have not had any previous treatment. There are other trials that require patients to allow a period of time before switching from a standard treatment to a study treatment. Some trials exclude patients with illnesses such as liver or kidney disease because the study uses treatments that put stress on these body parts.

Some patients may be concerned about participating in a clinical trial because of

- Fear of getting less than standard care/receiving a placebo (see *page 14*)
- Language barriers or cultural differences
- Treatment-related expenses (see *page 7*)
- Lack of access to trials at a clinic/institution (see *page 6*)
- Family/work responsibilities (see *page 9*).

If you are interested in a clinical trial, speak to the doctor or nurse on your treatment team. He or she will answer any questions you have and discuss your concerns.

How to Find Cancer Clinical Trials That May Be Right for You

There are thousands of open cancer clinical trials across the United States, and finding a clinical trial can be overwhelming.

As you begin your search, you will need to know certain details about your cancer diagnosis. It is important to know

- Your type of cancer
- The stage of cancer
- What treatments (and responses) you have already had.

This will be helpful information when you start reviewing the eligibility criteria of various clinical trials.

It is important to speak to your doctor or another member of your health care team about clinical trials. They may know about a clinical trial that would be a good option for you.

You can also work one on one with an LLS Clinical Trial Nurse Navigator who will assist you throughout the entire clinical-trial process. Our Clinical Trial Nurse Navigators are registered nurses with expertise in blood cancers, clinical trials and bone marrow transplant. Your Clinical Trial Nurse Navigator will

- Provide you with a detailed list of appropriate clinical trials to discuss with members of your healthcare team
- Provide education about clinical trials and your disease journey
- Speak with you to understand your goals, provide guidance and help you to navigate the clinical-trial process
- Help you to understand the clinical-trial process, including your rights and obligations as a patient
- Ask you for details about your diagnosis, including your genetic profile, past treatments and responses, your current physical condition and medical history that might impact your eligibility for certain clinical trials
- Help you to understand how your financial situation, insurance coverage, support network and ability and willingness to travel far distances might impact your choice of clinical trials
- Guide and advocate for you in your efforts to enroll in a clinical trial, including connecting you with trial sites
- Help address and overcome obstacles to enrollment
- Be available for support throughout your experience in the clinical-trial process.

Please call an LLS Information Specialist at (800) 955-4572 or visit www.LLS.org/CTSC for more information about this program.

Deciding to Take Part in a Clinical Trial

The decision to participate in a clinical trial is a very personal one. The choice to participate in a clinical trial is yours and yours alone, although you will want input from those close to you and members of your healthcare team. You will need to weigh the possible risks versus the possible benefits of participating in a clinical trial. You will also need to consider how you will pay for the costs of your care and how your participation in the clinical trial will affect the day-to-day life for you and your family members.

Weighing the Risks and Benefits. As with any treatment option, a clinical trial has possible benefits, as well as risks. You should learn about the benefits and risks of a clinical trial before you agree to participate in it.

Possible benefits of participating in a clinical trial include

- Early access to a promising new treatment that may not be available outside of a clinical-trial protocol (detailed plan of the study)
- High-quality care from members of the trial’s healthcare team, including close monitoring of your disease and side effects of treatment
- The possibility that some or all of the costs of the study treatment will be paid for by the manufacturer of the study drug
- Helping to advance cancer research (your participation contributes to the body of knowledge that will help other cancer patients, now and in the future)
- Access to doctors with extensive experience in treating your type of cancer.

Possible risks of participating in a clinical trial may include

- Experiencing unknown side effects of the new treatment (different than the side effects of existing treatments)
- The possibility that the new drug or the new treatment will be ineffective
- More frequent visits to the doctor for additional tests and examinations
- The possibility that your insurance policy may not pay all, part or any of the costs of the study.

Costs and Insurance. Before you agree to participate in a clinical trial, it is important to inquire about insurance coverage and gather other financial information.

The following three types of costs are associated with a clinical trial:

- 1) Research study costs—The costs that cover the clinical-trial-related treatment(s). These costs may include the investigational intervention (such as the drug being tested), extra doctor’s visits, and laboratory and imaging tests done solely for research purposes. Often, the trial sponsor will cover such costs.
- 2) Routine medical care—These costs are related to your cancer treatment, whether or not you are a patient in a clinical trial. These costs may include, doctor visits, hospital stays, laboratory and imaging tests, standard care cancer treatments, and treatments to either reduce or eliminate cancer symptoms or alleviate the side effects of treatment. These costs would be billed to your health insurance. The Affordable Care Act (ACA) requires most private health insurance plans to cover the routine medical costs of care.

3) Out-of-pocket expenses—These are the additional patient costs; they include travel expenses (gas, wear on the car, plane/train tickets), food while at the treatment center, lodging, etc. These costs are not covered by insurance.

Insurance companies cannot drop your coverage or refuse to let you take part in a clinical trial. In some cases, clinical trials may require additional screenings and tests that may not be covered by your insurance company, however sponsors of clinical trials will often cover these costs if it is needed for the study.

Insurance companies are not required to cover care from out-of-network providers or hospitals. To avoid any surprise out-of-pocket costs associated with a clinical trial, you should talk with your health care team and your insurance company directly.

Visit Triage Cancer at www.triagecancer.org to follow any changes related to insurance coverage for a clinical trial.

Medicaid Coverage. Each state has its own plans for patients covered by its Medicaid program. Please check to see if your state covers any routine patient care costs incurred during clinical trials.

Medicare Coverage. If you have Medicare health insurance, Medicare may pay for many of the routine medical costs for patients with cancer who are participating in approved clinical trials. If you are unsure about whether your trial meets Medicare's requirements, discuss your concerns with the research team and visit Medicare.gov, or call 1-800-MEDICARE (1-800-633-4227) TTY users should call 1-877-486-2048.

Insurance Benefits. In order to know whether your treatment will be covered, you must be knowledgeable about your health insurance benefits. It is important to speak to a member of the trial team and your insurance provider to learn ahead of time what costs will be covered. There are steps that you can take to find out about your coverage. Insurance reimbursement varies depending on the treatment, the insurance company and the health insurance policy. Request a written explanation of your benefits from your insurance company to understand what is covered prior to starting a clinical trial. If you are denied coverage, it may help to have your doctor either talk to a representative at your health insurance company or file a written appeal with the health plan's representative or medical director. The LLS Information Specialists and Clinical Trial Support Center personnel can help you to navigate these barriers. Please call (800) 955-4572 to speak to an Information Specialist or a Nurse Navigator who can assist you.

How Participation in a Trial May Affect You and Your Family. Before enrolling in a clinical trial, patients may want to consider how the trial will affect their quality of life, such as treatment side effects, time commitment and travel requirements. Different clinical trials require different levels of participation by patients. For example, a trial that is done on an outpatient basis may have a very different impact on a patient's lifestyle than a trial that requires hospitalization. Some trials may require follow-up tests that are time consuming and/or invasive. Factors, such as these, may affect a person's desire and ability to participate in a clinical trial.

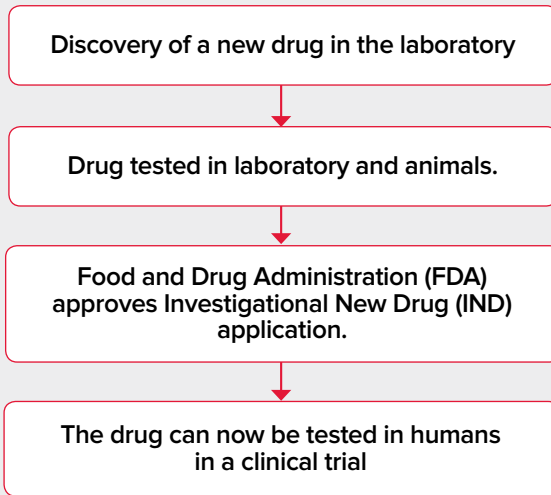
Additionally, financial difficulties can be overwhelming. Clinical trials may involve frequent trips to the hospital, requiring time off from work and expenses for travel, parking and childcare. LLS has a number of financial assistance programs that may be able to help. Visit www.LLS.org/finances or call (800) 955-4572 for more information and see *Resources From Other Organizations* on page 25 for additional organizations that may be able to assist patients with these expenses.

How Is a Clinical Trial Planned and Organized?

Preclinical trials (early research) and clinical trials are implemented so that researchers can evaluate newly discovered drugs and new approaches to therapies and drug regimens. See **Figure 1** on page 10. If a new drug is discovered, a preclinical trial is initiated and the drug is developed and tested in a laboratory setting. When the preclinical trial process demonstrates that the therapy is safe and effective, the Food and Drug Administration (FDA) needs to approve an investigational new drug (IND) application. Once that approval is in place, a monitored clinical trial is instituted. The clinical trial is a phased protocol with designated end points (goals). It is planned, organized and then documented in precise detail.

A clinical trial may also be initiated so that researchers can investigate whether drugs that are already approved by the FDA for specific blood cancers are effective in the treatment of other blood cancers too. In other clinical trials, researchers explore new combinations of drugs that have the potential to offer even more therapeutic options.

Figure 1. New Drug Development



Clinical Trials for Cancer Drugs

A clinical trial for a cancer drug is developed and led by experienced doctors who specialize in cancer research. They decide on

- The disease to be treated
- The treatment that will be tested
- The goal(s), sometimes called “end point(s),” of the study
- The type of patient who will be an appropriate participant in the study
- Ways to protect the patient’s safety
- Drug dosages or whether other treatments will be given to patients in the trial
- How long the treatment will be studied in the trial.

The Phases of Clinical Trials for Blood Cancers

For a drug to become a standard treatment that is widely accepted by medical experts, it must be tested in clinical trials and go through a series of steps called “phases.” See **Figure 2**, *below*. Typically, a cancer drug clinical trial is divided into four phases. Each phase is designed to answer certain questions. As each phase of the trial is successfully completed, the investigation of the drug may move into the next phase.

In the early phases, researchers determine whether the treatment is safe and effective. In later phases, they evaluate the treatment delivered in the clinical trial to see if the investigational new therapy works better than the standard treatment. In all phases, researchers watch patients closely for possible side effects of the treatment and monitor patient safety. See **Table 1** on *page 12*.

Figure 2. How Do Clinical Trials Work?

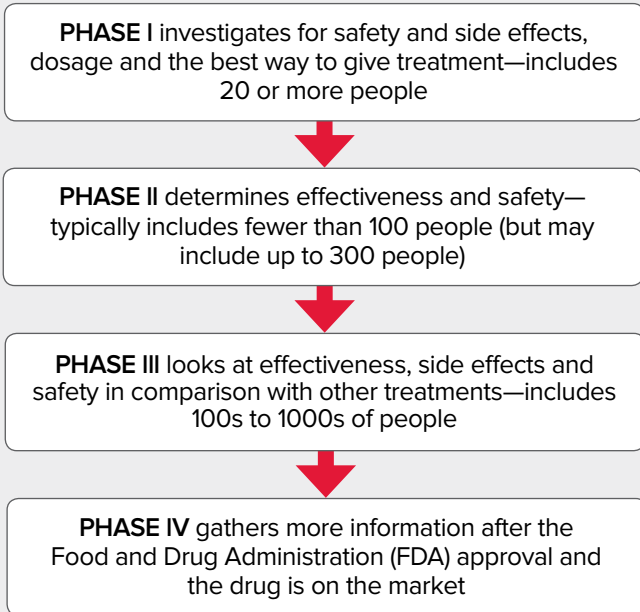


Table 1. Phases of Clinical Trials

Phase I

The purpose of the study is to

- Test the safety of the treatment
- Determine the maximum tolerated dosage/therapeutic dosage
- Learn how the treatment affects the cancer and the side effects of that treatment

Information About Phase 1

- Small group of patients enroll (20 or more)
- This could be the first time a drug or combination of drugs is tested in humans; called “first in human”
- This is the first time a drug is studied as a treatment for a particular condition.
- A drug already approved by the Food and Drug Administration (FDA) and in use for treating one disease, can be tested and evaluated for the first time in a different disease.
- This is the first time a treatment administered in a particular regimen (for example, in a new combination with other drugs) can be evaluated.

Phase II

The purpose of the study is

- To test the effectiveness and identify side effects.

Information About Phase II

- Up to 300 patients enroll
- This is the first time that doctors and researchers will be looking to see whether the new drug treatment works for a specific cancer.
- If the phase II trial shows that the treatment is safe and likely to work, the drug treatment could be submitted for FDA approval.
- If researchers are trying to compare the effects of the new drug or treatment approach to the outcomes of standard treatment, further research may be undertaken under phase III trial protocols. Research can be moved to a phase III trial right away or at a later date.

Phase III

The purpose of the study is

- To look at effectiveness, side effects and determine whether the drug is beneficial to the specific disease population.

Information About Phase III

- Between 300 and several thousand patients enroll
- Phase III trials are structured so that researchers can determine whether the study treatment is more effective than standard treatment, has fewer side effects, or both.
- A phase III trial looks at a number of different patient groups (referred to as “arms”). Each patient group is associated with a specific drug treatment regimen—a different treatment regimen is given to each group. Assigning people to different groups is called “randomization.”
- Phase III randomized trials are designed to be “single-blind,” “double-blind,” or “open label” studies.
 - In a single-blind study, patients do not know whether they are receiving the study treatment or the standard treatment.
 - In a double-blind study, neither the patients nor the clinicians (doctors and/or nurses) treating the patients know which participants are receiving what treatment. A double-blinded randomized study is considered the gold standard of scientific evidence for a new treatment because this type of study minimizes the possibility of unintended biases.
 - In an open label clinical trial, both the patient and the doctor and/or nurse will know what treatment the patient is getting.
 - Not knowing which treatment group they have been assigned to can be difficult for patients; however, blinded studies reduce the risk that doctors will be biased in their evaluations of patient outcomes.
- After the phase III trial is completed, the FDA reviews the trial results. The FDA will then approve the new treatment if it is
 - More effective than standard treatment
 - Equally effective as standard treatment but has less toxic side effects.

Phase IV

The purpose of the study is

- To gather long-term data on safety and effectiveness after FDA approval and the drug is on the market.

Information About Phase IV

- Several thousand patients enroll

Some cancer clinical trials are funded by an institution, such as the National Cancer Institute (NCI). Others are funded by organizations or industry—pharmaceutical companies, for example. A trial may take place at just a few specific locations or it may be offered at large cancers centers, university hospitals or local medical centers across the United States or the world.

Common Concerns

Will I get a placebo?

A placebo is a pill, liquid or powder that looks like the drug being used in the treatment regimen but it does not have any effect on the disease. The placebo is inactive. **Placebos are not used in cancer clinical trials unless they are given along with an active drug.**

In some clinical trials, researchers want to learn if adding a new drug to the standard therapy makes the treatment more effective. In these studies, some patients get the standard treatment and the trial drug, while other patients get the standard treatment and a placebo. It is unethical to give someone a placebo if there is a treatment available that could work. If a placebo is part of the treatment in a clinical trial, you will be told in advance and your doctor will discuss the drug regimen in further detail with you.

Will I be a guinea pig?

The fear of having no control is a common concern among patients. The idea that a trial patient is being used as a guinea pig is very misleading. It implies that the patient is at the mercy of researchers and may be experimented on without his or her consent. This is not the case. Clinical trials are carefully designed studies that put the health and safety of patients first. Before you agree to participate in a clinical trial, you will be taken through the informed consent process (See *The Informed Consent Process* on page 16). This process gives you the opportunity to obtain information about the study and ask questions. Only by signing the informed consent, are you deciding that you want to join the study. No one can be forced to take part in a study. Participation in a clinical trial is always voluntary, and patients can leave the study at any time.

Can I pick which treatment group I will be in?

If you are in a randomized study, you will not be able to choose your treatment group. Randomization gives each patient an equal chance of being assigned to any of the groups. Some patients find the idea of not being able to choose their treatment group distressing especially if they want to be in the group receiving the study treatment. It is important to remember, however, that

- Every patient in a clinical trial can expect to receive excellent medical care, regardless of whether he or she receives the new treatment or the standard treatment.

- It is not known whether the study treatment is actually better than, the same as, or less effective than the standard treatment.

Are Clinical Trials Safe?

A clinical trial is designed to gather scientific information about a treatment, but the safety of individual patients is the highest concern. All trials follow strict scientific and ethical principles. Every trial has a person in charge, usually a doctor, called the “principal investigator.” The principal investigator must develop a study plan called a “protocol” (See *Study Protocol, below*). The protocol is carefully designed to provide answers to specific research questions while balancing the potential benefits and risks to patients. The same protocol is used by every doctor at every treatment center taking part in the trial.

Study Protocol. A study protocol is described in a document that details the background, rationale, objectives, design, methodology, statistical considerations, and organization of a clinical research project. Documents describing a protocol can be very long (sometimes 100 pages or more) and they can be very technical. A protocol document is used by the investigator and members of the research team. The patient does not see this document. Instead, all of the important information about the trial will be included in the informed consent and available to the patient in that way.

The protocol specifies

- The purpose of the study
- The phase of the study
- The number of patients who will be recruited for the study
- The profile of the type of patients eligible to participate in the study (eg, patients with a particular type of blood cancer, meeting general health requirements, etc)
- Whether the trial is blinded or open label
- The treatment dosage, frequency of administration and how the treatment is given (as a pill, an infusion, or subcutaneously, etc)
- The medical tests patients will be asked to undergo and how often they will be required
- Protections against risks to patients
- The number of visits that will be required for follow up (either at the study location or with a local doctor)
- The type of personal information that will be collected about the patient and procedures to protect patient confidentiality
- The length of the study.

Eligibility Criteria. Each clinical trial protocol includes guidelines called “eligibility criteria” that state which patients are eligible to participate in the study. Not everyone who is interested in a clinical trial and would like to enroll will be accepted. Each trial can only accept patients into the study who meet the eligibility criteria. For instance, some studies are looking for patients who have a certain type and stage of cancer. Characteristics that patients must have to be eligible for a study are called “inclusion criteria.”

There are also some factors that can exclude a person from a study. For example, a study may be only seeking patients in a certain age-group, so a patient who is older or younger than the age range of the specified age-group would not be able to participate in the study. Factors that exclude a patient from taking part are called “exclusion criteria.”

Common inclusion and exclusion criteria include

- Type of cancer
- Stage of cancer
- Specific genetic markers or mutations
- A history of another cancer
- Specific laboratory test results
- Treatment history
- Age
- Certain medications a person is taking
- Current health status.

These criteria help to ensure

- Safety: Some patients may have other (coexisting) health problems that could be made worse by the treatments tested in the study.
- Accurate study results: Eligibility criteria ensure that new approaches are tested on similar groups of patients. This way, doctors can be sure that the results are attributable to the treatment being studied and not to other factors.

The Informed Consent Process. Federal regulations and guidelines require researchers to give potential patients complete and accurate information about a clinical trial. “Informed consent” is the name given to the ongoing process in which a research team member explains the details of the study to the patient and answers any/all questions and concerns about the clinical trial.

If you are considering participating in a clinical trial, the doctors and nurses involved in the trial will explain the study to help you decide if you want to take part. You, as well as your caregivers, should be present. If you need the services of a language interpreter you can request one.

The informed consent process gives you

- A chance to ask questions—both during your first meeting and then at follow-up meetings—so you have the information you need to make a decision.
- Time to review the details of the study; you will be given written information so that you can take it home, read it over, and discuss it with your doctor, family or others you trust.
- Information about what to expect in the study (tests, financial burdens, option to decline at any time).

You must agree to sign an informed consent document before you can begin the trial protocol. It affirms that you fully understand the nature of the study. It is not a contract, though, and you are free to leave the study if any new information leads you to want to do so. In fact, you may withdraw from the trial at any time, for any reason. If you do enter a study and then withdraw you should let the research team know your reasons for leaving the study.

The informed consent document includes

- Details about the study, such as its purpose and length (duration)
- Key contacts
- The examinations and laboratory tests that are needed
- Study risks and potential benefits.

You can decide whether or not to sign the informed consent document after reading it. Take the time you need to think about it and discuss it with family members or caregivers. Be sure that you fully understand the information. Read the informed consent document very carefully. If you have questions and concerns, write them down. Take that list with you to your next appointment with the doctor or nurse on the research team. With a list, you will be sure to remember what issues you wanted to address and get answers to all your questions.

New information may become available to the research team as the trial goes along. The clinical trial study design (protocol) may also change over time. The informed consent process requires that members of the research team update you when there are such changes. You may also be asked to sign a new informed consent document.

Informed Consent for Children. The safety of children remains the greatest priority in research studies, and children under legal age get special protection as research subjects. The vulnerable nature of children must be considered when balancing the risks of research with the need to develop safe and effective pediatric treatments.

By law, children who participate in a clinical trial must have the disease or condition being studied. Further, when researchers give an investigational drug to

children, they must provide evidence of potential clinical benefit that justifies the risk of taking the drug.

Almost always, both parents/guardians must give legal consent for their child to take part in a clinical trial. Children are not able to give true informed consent until they reach a legal age. Once patients are of legal age, they can sign their own consent forms.

The decision to participate in a clinical trial should be discussed with both the parents/guardians and the child. As a rule, researchers will ask children ages 7 and older if they agree to participate in the study. They are asked for their “assent” which means that they agree to take part. The age for assent, however, varies depending on the child and the institution running the trial. Not all children will desire an active role in the informed consent process, but it is important to give a child the opportunity to learn about the research and ask any questions he or she may have. It may take a few sessions before a child truly understands the concept of the research trial and before the research team feels that the child understands. At that point, the child can assent. There are times when assent may be waived, for example if the child is too ill to participate in the assent process.

Research teams generally do not ask children under the age of 7 for their assent since they are typically too young to understand the concept of taking part in a clinical study. If there is a difference in opinion between the child and the parents/guardians, sometimes an advocate, such as a psychologist or social worker, can help with continued family discussions to help them make a unified decision.

As with the informed consent process, the assent process is meant to be an ongoing conversation between the child and members of the research team. During the assent process

- Parents/guardians give informed consent for their child to participate in the clinical trial.
- A member of the research team will explain the trial to the child in language that the child can understand. This explanation includes what it means to take part in a clinical trial and what to expect. The research team may use written forms, pictures and video to help explain the process.
- The research team encourages the child to ask questions.

Parents/guardians can take their child out of a research study at any time, for any reason. If the child is of legal age or older, he or she can decide to stop participating in the study at any time. If a child age 7 or older asks to stop participating in the trial, there should be a discussion with parents/guardians and the treatment team. If parents/guardians do plan to take the child out of a clinical trial, it is important to discuss other treatments with the child’s primary hematologist-oncologist.

Scientific Review. There are several procedures in place to ensure that clinical trials are conducted in a safe and ethical manner (See **Table 2, below**). Clinical trials have to go through different types of review that are designed to protect all patients who participate. These reviews are conducted by the sponsoring organization, the principal investigator, the Institutional Review Board (IRB), and the Data and Safety Monitoring Board (DSMB).

Table 2. Safeguards in Clinical Trials

<p>Oversight/monitoring provided by the sponsoring organization</p> <p>The sponsoring organization takes responsibility for initiation, management and financing of the study.</p>	<p>Responsibility of the sponsoring organization</p> <p>The sponsoring organization provides outside experts who will evaluate the purpose and design of the study. These experts will also determine if the study is of value and if it is possible, as designed, to conduct it safely in the setting(s) as described.</p>
<p>Oversight/monitoring provided by the study (principal) investigator</p> <p>The principal investigator supervises the treatment plan.</p>	<p>Responsibility of the principle investigator</p> <p>The principle investigator for the trial prepares or follows an action plan (protocol) for the study that details how many patients will take part in the study, what medical tests they will receive and how often, and the treatment plan.</p> <p>There may be many cooperative centers (participating institutions) in different locations offering the same clinical trial. The principle investigator ensures that the same protocol is used by each doctor who takes part in the study.</p>

Continued ...

Table 2. Safeguards in Clinical Trials (Continued)

Oversight/monitoring provided by the Institutional Review Board (IRB)

Each participating institution has an IRB or uses a centralized IRB, made up of healthcare professionals, scientists and community members. The IRB's purpose is to protect patients who take part in clinical trials. The IRB ensures that the clinical trial is legal, ethical, well designed and does not involve unneeded risks.

The IRB reviews, monitors and approves the treatment plan for a clinical trial. The IRB is charged with protecting the interests and welfare of patients who take part in a clinical trial.



Responsibility of the IRB

The IRB

- Reviews the protocol to make sure the study is conducted fairly and safely
- Decides how often to review the trial once it has begun. Based on this information, the IRB decides whether the clinical trial should continue as initially planned and, if not, what changes should be made
- Can stop a clinical trial if the researcher is not following the protocol or if the trial appears to be causing unexpected harm
- Can stop a clinical trial if there is clear evidence that the new drug/treatment is effective, in order to initiate steps to make it widely available.

Oversight/monitoring provided by the Data and Safety Monitoring Board (DSMB)

The DSMB is an independent committee made up of statisticians, physicians and other expert scientists. It monitors data from a clinical trial for safety problems.



Responsibility of the DSMB

The DSMB

- Periodically monitors the tests, results, and safety of trials to ensure that the risks of participation are as low as possible
- Makes sure the data are complete
- Monitors all trial results; if early results show clear advantages of a new drug, the DSMB can recommend that the trial should end early to move on to the approval process more quickly. The DSMB might also recommend that a trial should end early if there is compelling evidence that the new treatment is not working or that it has severe or life-threatening side effects.

What if I Am Not Eligible for a Clinical Trial?

If you are not eligible for a trial, there may be other ways to gain access to treatments that are not approved by the Food and Drug Administration (FDA) for the treatment of your diagnosis. Expanded access (sometimes called “compassionate use”) may provide an opportunity for patients to gain access to investigational drugs outside of a clinical trial when no satisfactory alternative therapy is an option. Expanded access may be allowed when all of the following criteria apply:

- The patient has a serious disease or his or her life is immediately threatened by disease.
- There is no comparable or satisfactory alternative therapy to treat the disease.
- The patient cannot enroll in a clinical trial.
- Potential benefit to the patient justifies the potential risks of treatment.
- Providing the investigational treatment will not interfere with the investigational trials.

Please visit <https://www.fda.gov/forpatients/other/default.htm> for up-to-date information about all of the ways a patient can access a treatment outside of a clinical trial (such as compassionate use [expanded access] or off-label use).

Feedback. Please visit www.LLS.org/PublicationFeedback to make suggestions about the content of this booklet.

Resources and Information

LLS offers free information and services to patients and families affected by blood cancer. This section of the booklet lists various resources that can be helpful to you. Use this information to learn more, to ask questions, and to make the most of your healthcare team members' knowledge and skills.

For Help and Information

Consult with an Information Specialist. Information Specialists are master's level oncology social workers, nurses and health educators. They offer up-to-date information about disease, treatment and support. Language services are available. For more information, please

- Call: (800) 955-4572 (Monday through Friday, 9 am to 9 pm ET)
- Email: infocenter@LLS.org
- Live chat: www.LLS.org/InformationSpecialists
- Visit: www.LLS.org/InformationSpecialists.

Clinical Trials Support Center. Clinical trials for new treatments are ongoing. Patients and healthcare professionals can learn about clinical trials and how to access them. When appropriate, personalized clinical-trial navigation by registered nurses is also available through the Clinical Trial Support Center. For more information, please

- Call: (800) 955-4572 to speak with our LLS Information Specialists
- Visit: www.LLS.org/CTSC.

Free Information Booklets. LLS offers free education and support booklets that can either be read online or ordered. Please visit www.LLS.org/booklets for more information.

Telephone/Web Education Programs. LLS offers free telephone/Web and video education programs for patients, caregivers and healthcare professionals. Please visit www.LLS.org/programs for more information.

Financial Assistance. LLS offers financial assistance to individuals who have blood cancer. Please visit www.LLS.org/finances for more information.

Co-Pay Assistance Program. LLS offers insurance premium and medication co-pay assistance for certain eligible patients. For more information, please

- Call: (877) 557-2672
- Visit: www.LLS.org/copay.

One-on-One Nutrition Consultations. Access free one-on-one nutrition consultations provided by a registered dietitian who has experience in oncology nutrition. Dietitians assist callers with information about healthy eating strategies, side effect management, and survivorship nutrition. They also provide additional nutrition resources. Please visit www.LLS.org/nutrition for more information.

Podcast. Listen in as experts and patients guide listeners in understanding diagnosis, treatment, and resources available to blood cancer patients. *The Bloodline with LLS* is here to remind you that after a diagnosis comes hope. Visit www.LLS.org/TheBloodline for more information and to subscribe.

Suggested Reading. LLS provides a list of selected books recommended for patients, caregivers, children and teens. Please visit www.LLS.org/SuggestedReading to find out more.

Community Resources and Networking

LLS Community. The one-stop virtual meeting place for talking with other patients and keeping up to date with the latest blood cancer resources and information. Share your experiences with other patients and caregivers and get personalized support from trained LLS staff. Please visit www.LLS.org/community to join.

Weekly Online Chats. Moderated online chats can provide support and help cancer patients to reach out and share information. Please visit www.LLS.org/chat for more information.

LLS Chapters. LLS offers community support and services in the United States and Canada including the *Patti Robinson Kaufmann First Connection Program* (a peer-to-peer support program), in-person support groups and other great resources. For more information about these programs or to contact your chapter, please

- Call: (800) 955-4572
- Visit: www.LLS.org/ChapterFind.

Other Helpful LLS Resources. LLS offers an extensive list of resources for patients and families. There are resources that provide help with financial assistance, counseling, transportation, patient care and other needs. For more information, please visit www.LLS.org/ResourceDirectory to obtain our directory.

Advocacy. The LLS Office of Public Policy (OPP) enlists volunteers to advocate for policies and laws to speed new treatments and improve access to quality medical care. For more information, please

- Call: (800) 955-4572
- Visit: www.LLS.org/Advocacy.

Additional Help for Specific Populations

Información en Español (LLS information in Spanish). Please visit www.LLS.org/espanol for more information.

Language Services. Let members of your healthcare team know if you need a language interpreter or other helper, such as a sign language interpreter. Often, these services are free.

Information for Veterans. Veterans who were exposed to Agent Orange while serving in Vietnam may be able to get help from the United States Department of Veterans Affairs. For more information, please visit www.publichealth.va.gov/exposures/agentorange or call the Department of Veterans Affairs at (877) 222-8387.

World Trade Center Survivors. People involved in the aftermath of the 9/11 attacks and subsequently diagnosed with a blood cancer may be able to get help from the World Trade Center (WTC) Health Program. People eligible for help include

- Responders
- Workers and volunteers who helped with rescue, recovery and cleanup at the WTC-related sites in New York City (NYC)
- Survivors who were in the NYC disaster area, lived, worked or were in school in the area
- Responders to the Pentagon and the Shanksville, PA crashes.

For more information, please

- Call: WTC Health Program at (888) 982-4748
- Visit: www.cdc.gov/wtc/faq.html.

People Suffering From Depression. Treating depression has benefits for cancer patients. Seek medical advice if your mood does not improve over time, for example, if you feel depressed every day for a 2-week period. For more information, please

- Call: The National Institute of Mental Health (NIMH) at (866) 615-6464
- Visit: NIMH at www.nimh.nih.gov. Enter “depression” in the search box

Resources From Other Organizations

The Drs. Jeffrey and Isabel Chell Clinical Trials Travel Grant

Email: patientgrants@nmdp.org

Call: (888) 814-8610

Visit: <https://www.jasoncarterclinicaltrialsprogram.org/education/grants>

The Drs. Jeffrey and Isabel Chell Clinical Trials Travel Grant in partnership with the Jason Carter Clinical Trials Program, provides financial help to qualified patients (those with blood disorders/cancers), who need help paying the following costs to travel for clinical trials:

- Patient and companion air travel: Booked by dedicated travel agents
- Ground transportation: Gas/parking and public/mass transit (bus/train/cabs etc.)
- Accommodations: Hotel, temporary housing and incidentals.

Food and Drug Administration (FDA)

Call: (888) INFO-FDA or (888) 463 6332

Visit: FDA at www.fda.gov

An agency in the US federal government that ensures that drugs, medical devices and equipment are safe and effective.

Lazarex Cancer Foundation

Call: (925) 820-4517

Visit: www.lazarex.org

The Lazarex Cancer Foundation provides financial assistance directly to patients for costs associated with clinical-trial participation, and offers navigation to help patients identify their FDA clinical trial options.

The Foundation can help with the following costs associated with participating in an FDA-approved cancer clinical trial:

- Transportation (airfare, gas, rental cars, taxi fare, parking/tolls)
- Lodging (short-term and long-term housing)
- Certain medical expenses not covered by insurance and necessary for clinical-trial treatment.
- Funding for a travel companion to provide emotional and logistical support while a patient is in treatment, away from home, family and friends.

My Healthcare Team Contact List

Use this list to remember names and contact information for members of your healthcare team.

CAREGIVER NAME:

Address: _____

Phone Number/Fax number: _____

Email address: _____

Additional information: _____

PRIMARY CARE PROVIDER (PCP):

Address: _____

Phone Number/Fax number: _____

Email address: _____

Additional information: _____

PHARMACY NAME:

Address: _____

Phone number/Fax number: _____

Additional information: _____

Information Specialists:

Phone: 1-800-955-4572

Email: infocenter@LLS.org

Website: www.LLS.org/InformationSpecialists

HEMATOLOGIST-ONCOLOGIST NAME:

Address: _____

Phone number/Fax number: _____

Email address: _____

Website/Portal: _____

Additional information: _____

NURSE/NURSE PRACTITIONER (NP) NAME:

Phone number/Fax number: _____

Email address: _____

Additional information: _____

RESEARCH NURSE NAME:

Address: _____

Phone number/Fax number: _____

Email address: _____

Additional information: _____

RESEARCH COORDINATOR NAME:

Address: _____

Phone number/Fax number: _____

Website or email address: _____

Additional information: _____

SOCIAL WORKER (SW) NAME:

Address: _____

Phone number/Fax number: _____

Email address: _____

Additional information: _____

INSURANCE CASE MANAGER NAME:

Address: _____

Phone number/Fax number: _____

Website or email address: _____

Additional information: _____

PHYSICIAN ASSISTANT (PA) NAME:

Address: _____

Phone number/Fax number: _____

Email address: _____

Additional information: _____

REGISTERED NURSE (RN) NAVIGATOR NAME:

Address: _____

Phone number/Fax number: _____

Email address: _____

Additional information: _____

OTHER:

Address: _____

Phone number/Fax number: _____

Email address: _____

Additional information: _____

OTHER:

Address: _____

Phone number/Fax number: _____

Email address: _____

Additional information: _____

OTHER:

Address: _____

Phone number/Fax number: _____

Email address: _____

Additional information: _____

OTHER:

Address: _____

Phone number/Fax number: _____

Email address: _____

Additional information: _____

Question Guides

Talk with members of the clinical trial team and ask questions. This will help you and your loved one to be actively involved in making decisions about medical care. You may want to ask members of your clinical trial team the following questions.

(Note: The use of “I (we)” and “me (us)” in lists of questions is used for situations in which the patient may either not be old enough or not able to make his or her own decision. A parent/guardian, relative, or caregiver may be either assisting the patient or making the decision.)

NAME: _____

Date of appointment or phone call: _____

Questions to Ask About Clinical Trials

1. What is the purpose of the clinical trial and why do researchers think the approach may be effective?
2. What kind of treatment does the study involve?
3. What is the phase of this study?
4. Has the treatment been studied before? Has it been used to treat other types of cancers?
5. What were the results in earlier studies/stages of this treatment?
6. Does this clinical trial include the use of a placebo?
7. How do the possible risks and benefits of this clinical trial compare with the risks and benefits of standard treatment?
8. What are the possible short-term and long-term side effects of both the standard treatments and the new treatment being tested in this clinical trial?
9. What happens if my health declines during the clinical trial?
10. How long will I be treated in the study?
11. Where is the clinical trial taking place? Is the clinical trial being offered at other sites?
12. Can I talk to other patients in the study?

13. How will I know if the study treatment is working for me?
14. Will I have responsibilities such as keeping a log or filling out forms about my health?
15. Who will know that I am taking part in the clinical trial? How will my confidentiality be protected?

Questions to Ask About Finances

1. How do I know whether my insurance will cover the costs of the clinical trial?
2. Will I have to pay for any part of the trial such as tests or the study drug?
3. Who can help answer any questions from my insurance company or health plan?
4. Will there be any travel or child care costs that I need to consider while I am in the trial?

Questions to Ask About Follow-up Care

1. If the treatment is effective, can I continue the treatment after the study has ended?
2. Will general results of the trial be provided to me?
3. Will the study team continue to check on me after the treatment is over?
4. Will I have to travel to the site for follow-up care or can it be done locally?



Get support.

Reach out to our

INFORMATION SPECIALISTS

The Leukemia & Lymphoma Society team consists of master's level oncology social workers, nurses and health educators who are available by phone Monday through Friday, 9 a.m. to 9 p.m. (ET).

- Get one-on-one personalized support and information about blood cancers
- Know the questions to ask your doctor
- Discuss financial resources
- Receive individual clinical-trial searches



Contact us at

800-955-4572 or

**[www.LLS.org/
informationspecialists](http://www.LLS.org/information specialists)**

(Language interpreters can be requested)



For more information, please
contact our Information Specialists
800.955.4572 (Language interpreters
available upon request).

**BEATING
CANCER
IS IN
OUR BLOOD.**

National Office 3 International Drive, Suite 200 Rye Brook, NY 10573

The mission of The Leukemia & Lymphoma Society (LLS) is to cure leukemia, lymphoma, Hodgkin's disease and myeloma, and improve the quality of life of patients and their families. Find out more at www.LLS.org.