LYMPHOMA
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Getting the Facts

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Understanding Clinical Trials

Overview

A *clinical trial* (also called a clinical study) is a carefully designed research study conducted to answer specific questions about new ways to prevent, diagnose, treat, or manage a disease or the side effects caused by a new or existing treatment. Participation in a clinical trial contributes to medical knowledge. The investigators in clinical trials want to determine the safety and effectiveness of the treatment being studied by making specific assessments before, during, and after the trial. Strict rules and oversight procedures make sure that clinical trials are designed and run in a way that protects the rights and safety of the people who volunteer to participate.

Some common reasons for conducting clinical trials include:

- Evaluating one or more products/interventions (e.g., drugs, medical devices, or approaches to surgery or radiation therapy) for treating a condition, disease, or syndrome
- Finding ways to prevent the initial development or recurrence of a disease or condition, including medications, vaccines, or lifestyle changes such as diet
- Evaluating one or more interventions aimed at identifying or diagnosing a particular disease or condition or identifying a risk factor for that condition
- Exploring and measuring ways to improve the comfort and quality of life of people with a chronic illness through supportive care

As shown in Table 1 below, there are four main types or phases of clinical trials. The first three (Phase I, Phase II, and Phase III) are usually required before a drug is considered for approval by the U.S. Food and Drug Administration (FDA). Phase IV trials, sometimes called postmarketing studies, are conducted after a drug has received FDA approval. Each phase is designed to find out certain information, building upon the knowledge learned from the previous phase. Patients may be able to participate in different trials depending on their health status, their type and stage of cancer, and the types of treatments, if any, that they have previously received.

Participating in a Clinical Trial

Clinical trials are not a "last resort" for patients. Every drug available today had to be tested in clinical trials before it was approved for general use, and all new and emerging treatments for lymphoma and chronic lymphocytic leukemia/small lymphocytic lymphoma (CLL/SLL) must be tested this way before patients can use them in the future. Participating in a clinical trial can help to improve treatment options for patients for many years to come. Clinical trials offer both benefits and risks. Patients in clinical trials may be able to try new treatments that are not otherwise available to all patients. However, this new treatment may or may not be more effective than the standard therapy. At the very least, patients who are randomized to the control group will receive the standard therapy that they would have received if they had not enrolled in the trial. The health of the patient enrolled in a clinical trial is monitored very carefully. The healthcare team studying the new treatment will explain all of the possible risks and benefits of a specific clinical trial.

Every clinical trial is led by a principal investigator, who is usually a medical doctor. Clinical trials also have a research team that may include nurses, physician assistants, social workers, medical coordinators, and other healthcare professionals. Patients usually continue regular visits with their current healthcare provider, who may work with the research team to ensure that any investigational treatment will not interfere with current medication or treatments. Clinical trials are carefully supported by safety monitoring boards, monitoring processes, audits, and other activities to ensure ongoing safety assessments. Participants join clinical trials on a volunteer-basis.

Informed Consent in a Clinical Trial

Informed consent is the process in which patients learn about all of the expected risks, potential benefits, and alternatives of the clinical trial they are considering. After the healthcare team has explained everything and answered all questions, patients are asked to read and sign an informed consent before entering the study that details all the trial information discussed, describes how their records will be kept private, and shows that he or she was given information on the

Table 1. The Four Phases of Clinical Trials

Phase	Purpose	Typical Number of Volunteer Patients
Phase I	• To identify a safe dose of a new drug • To decide on a dosing schedule for the drug • To see what side effects are related to the therapy	• 15–30 patients with one or more different types of cancer
Phase II	• To see if a new treatment is effective against a certain type of cancer at the dose determined in Phase I • To confirm and learn more about the side effects identified in Phase I	• Usually less than 100 patients with the same type of cancer • More than 100 people in two study arms for randomized Phase II studies
Phase III	• To compare the new treatment or new use of an existing treatment with the current standard treatments • To obtain detailed information about how well the treatment works and the types and severity of side effects it causes	• From 100 to several thousand patients with the same type of cancer • Patients are randomly assigned to a treatment group; one group receives the standard therapy, and the other group receives the experimental treatment
Phase IV	• To find out more information about the long-term safety and effectiveness of a new treatment after it has already been approved by the FDA and is being used by patients outside of a clinical trial	• Several hundred to several thousand people with the same type of cancer

risks, potential benefits, and alternatives. Patients can withdraw their consent and leave a clinical trial at any time. For patients who leave a trial or decide not to take part, their doctor will discuss the other treatment options available to them.

Use of Placebos in Phase III Studies

A *placebo* is an inactive ingredient that is given to patients in the control group of some clinical trials. The placebo is made to look and taste the same as the experimental pill, or to have the same appearance as the experimental intravenous or subcutaneous agent, so that patients cannot tell whether they have been randomized to the control group receiving the placebo or the experimental group receiving the new treatment. **In cancer clinical trials, patients are never given a placebo in place of an effective standard therapy.**

Questions to Ask About Clinical Trials

Here are some questions patients and their loved ones may want to ask in addition to understanding the potential benefits and risks of a clinical trial:

- What is the purpose of this clinical trial?
- Why are you recommending this clinical trial for me?
- Who is sponsoring this trial (the National Cancer Institute [NCI], a cancer center, an international study group, another state or national study group, or a pharmaceutical/biotechnology company)?
- Who has reviewed and approved this clinical trial?
- Does this clinical trial include the use of a *placebo* (a sugar pill or saline solution with no active ingredient/no intervention)?
- How long will the study last? Where will it take place?
- What are the risks involved?
- Do I always have to travel to the trial location in order to be monitored and to receive follow-up care?
- What are the possible benefits? If I benefit from the intervention, will I be allowed to continue receiving it after the trial ends?
- What are my responsibilities during the clinical trial?
- What kinds of tests, procedures, or treatments will be performed? How many and how often?
- Will I be in any discomfort or pain?
- Will I be able to see my own doctor during the clinical trial?
- What type of long-term follow-up care is part of this trial?
- What costs will I be responsible for? Who will pay for my participation? Will I be reimbursed for other expenses?
- What happens if my health gets worse during the clinical trial? Whom do I contact?

Cost Associated With Participating in a Clinical Trial

Clinical trials are very expensive undertakings for the study sponsor. However, the cost to the patient varies depending on the trial, who is sponsoring the trial, what portion of the study-related expenses the sponsor will cover, and the patient's health insurance coverage. According to the Affordable Care Act as of June 2017, health insurance plans issued after January 1, 2014, cannot limit or deny coverage for people who want to participate in approved clinical trials. Plans existing before this date may or may not provide coverage for the basic medical procedures associated with the trial, such as lab tests, scans, and hospitalization when required. Medicare provides coverage for patient care associated with most clinical trials. If a patient is taking part in an NCI trial being conducted at their campus in Bethesda, Maryland, the NCI will pay for the study drug and all costs related to the study, including assistance with travel, food, and lodging expenses. Some cancer centers provide financial assistance or discounted rates for room and meals and have special research units that will pay for study-related costs. There are also organizations that will provide financial assistance for treatment-related expenses. (For more information, please refer to the Lymphoma Research Foundation's [LRF's] *Resources for Financial Assistance* fact sheet.)

Finding Out About Lymphoma Clinical Trials

There are many ways to find out about clinical trials. Healthcare providers may be able to tell their patients about some clinical trials. Comprehensive cancer centers may also have information about clinical trials for different types of lymphoma. In addition, patients can contact the NCI's Cancer Information Center at (888) NCI-1937 or the NCI's Clinical Trials Referral Office at 800-4-CANCER. They can also search the National Institute of Health's websites (www.cancer.gov or www.clinicaltrials.gov) for user-friendly, comprehensive clinical trial listings and matching services for patients and professionals.

LRF Clinical Trials Information Service (CTIS)

LRF provides a "Clinical Trials Information Service" to increase awareness about trials being conducted at cancer treatment centers nationwide. Upon request, LRF Helpline staff can conduct a customized search for potential lymphoma treatment trials in a patient's area. Trial search results can be mailed or emailed so that they may be discussed with the patient's treating healthcare team and loved ones. Individuals interested in having a trial search conducted for them can contact the LRF Helpline at (800) 500-9976 or helpline@lymphoma.org.

Resources

LRF offers a wide range of resources that address treatment options, the latest research advances, and ways to cope with all aspects of lymphoma and CLL/SLL, including our award-winning mobile app. LRF also provides many educational activities, from in-person meetings to teleconferences and webcasts for people with lymphoma, as well as patient guides and e-updates that provide the latest disease-specific news and treatment options. To learn more about any of these resources, visit our website at www.lymphoma.org, or contact the LRF Helpline at (800) 500-9976 or helpline@lymphoma.org.

Contact the Lymphoma Research Foundation

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