Understanding Lymphoma and Chronic Lymphocytic Leukemia (CLL)

Clinical Trials

Lymphoma Research Foundation

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A clinical study involves research using human volunteers (participants) intended to contribute to medical knowledge. There are two main types of clinical studies: clinical trials (also called interventional studies) and observational studies.

In an observational study, investigators observe groups of participants or evaluate certain health outcomes according to a research protocol. Participants may receive interventions (which can include medical products such as drugs or devices) or procedures as part of their routine medical care, but participants are not assigned to a specific treatment or intervention by the investigator, as is the case in a clinical trial.

A clinical trial (also called an interventional 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. 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, on the following page, there are four main types or phases of clinical trials. The phase is based on the study's objective and the number of participants. 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 post marketing 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 n 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.

Table 1

Phases	Purpose	Typical Number of Volunteer Participants
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 look at long-term safety and effectiveness that take place after a new treatment has been approved by the FDA and is available to the public.	• From 100 to several thousand patients with the same type of cancer

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 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.

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 studyrelated 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 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.

Clinical Trials Information Service (CTIS)

The Foundation provides a "Clinical Trials Information Service" to increase awareness about trials being conducted at cancer treatment centers nationwide. Upon request, our 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 Helpline at **(800) 500-9976** or **helpline@lymphoma.org** or complete a trial search request form on our website at **www.lymphoma.org/ctis**.

Lymphoma Care Plan

Keeping your information in one location can help you feel more organized and in control. This also makes it easier to find information pertaining to your care and saves valuable time.
The Foundation's Lymphoma Care Plan document organizes information on your health care team, treatment regimen, and follow-up care. You can also keep track of health screenings and any symptoms you experience to discuss with your health care provider during future appointments. The *Lymphoma Care Plan* document can be accessed by visiting **lymphoma.org/publications**.

Patient Education Programs

The Foundation also offers a variety of educational activities, including live meetings and webinars for individuals looking to learn directly from lymphoma experts. These programs provide the lymphoma community with important information about the diagnosis and treatment of lymphoma, as well as information about clinical trials, research advances and how to manage/cope with the disease. These programs are designed to meet the needs of a lymphoma patient from the point of diagnosis through long-term survivorship. To view our schedule of upcoming programs, please visit **lymphoma.org/programs**.

Helpline

The Foundation's Helpline staff are available to answer your general questions about lymphoma and treatment information, as well as provide individual support and referrals to you and your loved ones. Callers may request the services of a language interpreter. The Foundation also offers a one-to-one peer support program called the Lymphoma Support Network and clinical trials information through our Clinical Trials Information Service. For more information about any of these resources, visit our website at **lymphoma.org**, or contact the Helpline at **(800) 500-9976** or **helpline@lymphoma.org**.

Para información en Español, por favor visite **lymphoma.org/es**. (For Information in Spanish please visit **lymphoma.org/es**).

Focus on Lymphoma Mobile App

Focus on Lymphoma is the first app to provide patients and their care partners with tailored content based on lymphoma subtype, and actionable tools to better manage diagnosis and treatment. Comprehensive lymphoma management, conveniently in one secure and easy-to-navigate app, no matter where you are on the care continuum. Get the right information, first, with resources from the entire Lymphoma Research Foundation content library, use unique tracking and reminder tools, and connect with a community of specialists and patients. To learn more about this resource, visit our website at **lymphoma.org/mobileapp**, or contact the Foundation's Helpline at **(800) 500-9976** or **helpline@lymphoma.org**.

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