A clinical trial is a controlled research study conducted by doctors and medical researchers to improve the care and treatment of people who have hematologic disorders or cancer. Taking part in a clinical trial may be the best treatment choice for some patients. There are trials for patients at every stage of treatment as well as those in remission. Virtually all of today’s standard treatments for hematologic conditions and cancer are based on previous clinical trials.

Results from research studies will help health-care professionals improve treatment options, increase patients’ survival, and improve patients’ quality of life. Read below for more information about how clinical trials work, the process for joining a clinical trial, and more.

This “Patient Education” tear sheet was produced in collaboration with The Leukemia & Lymphoma Society (lls.org).

How a Clinical Trial Works
Clinical trials are conducted under rigorous guidelines to determine safe and effective forms of treatment. Before a clinical trial begins, a new therapy is often developed and tested in a laboratory, then tested in animals. If the early research (preclinical trials) demonstrates that the therapy is safe and effective, a carefully planned and monitored clinical trial of the drug or treatment will then be conducted in humans.

Advances in treatment for blood cancers depend on clinical trials of new therapies or new therapy combinations. Different types of cancer clinical trials are designed to develop and test new and better ways to:

• diagnose and treat cancer in people
• prevent or relieve treatment side effects
• help prevent a return of cancer
• improve comfort and quality of life for people with cancer

Are Clinical Trials Safe?
Clinical trials are designed to give patients the safest, potentially most effective clinical therapies.

Patients enrolled in cancer clinical trials are never treated as “guinea pigs.” In fact, patients are given either the best treatment currently available or a new and possibly more effective therapy.

Patients in clinical trials are watched closely by their doctors, and by other members of their medical team, to ensure their safety. Patients receive a lot of attention and excellent cancer care. The trial can be changed or stopped if there is a problem. Patients who take part in a clinical trial also have the option to leave the trial at any time.

All clinical trials follow strict ethical principles with a detailed action plan (protocol) that specifies the:

• purpose of the study
• number of people who will be recruited
• group of patients eligible to participate in the study (e.g., patients with a particular type of hematologic cancer and general health requirements)
• treatments that participants will receive, including the dosage and dosing schedule (how often a drug will be administered)
• medical tests required
• number of follow-up visits required
• type of participant information that will be gathered
• event or outcome of the study that researchers will be able to measure (endpoint), such as response rates or time to progression of disease, toxicity, and quality of life.
Who Can Participate?
Depending on the study’s eligibility criteria (which outline what types of patients can and cannot participate), patients of any age, sex, or race, as well as patients at every stage of treatment (including those in remission) can be included. Researchers develop patient eligibility criteria based on disease type, patient demographics (e.g., age, sex, race), stage of disease, other treatments the patient has received, and the presence of any other illnesses or conditions.

Finding and Taking Part in a Clinical Trial
If you are interested in considering a clinical trial as a treatment option, first talk with your doctor, who can help you find a trial for which you may be eligible. If your doctor agrees that a clinical trial is a good option, he or she can contact the trial team to determine if the trial is right for you.

At times, you may need to contact the trial team yourself. When you contact a member of the trial team, ask to speak with the study coordinator, research nurse, the referral coordinator, or the protocol assistant. The study coordinator answers your and your doctor’s questions. He or she may be able to make a preliminary assessment of your eligibility for the trial. After your initial appointment with the coordinator, he or she decides whether you will be accepted to participate in the clinical trial.

Informed Consent
When you first express interest in a clinical trial, the doctors and nurses involved in the trial will describe the ongoing sharing of information by the trial team before, during, and after the clinical trial. This is known as informed consent. To start the process, the clinical trial team gives you an informed consent document with detailed written information about the trial, and the doctors and nurses involved in the trial can explain the study to help you decide if you want to participate.

This process is a chance for patients to:
• ask questions — both during your first meeting and then at follow-up meetings
• review the details of the study, which should be provided via written information so that you can take it home, read it over, and discuss it with your doctor, family, or others you trust
• understand what to expect in the study (e.g., tests, financial burdens, option to decline at any time)

Informed consent should also highlight details about the study, including its length, key contacts, examinations and laboratory tests required, and the potential risks and benefits. It is recommended that key decision-makers, including family members, be present for this information. Language services (i.e., an interpreter) may be requested and provided.

Next Steps
After your initial appointment with the coordinator, he or she will decide whether you are eligible to participate in the clinical trial. If so, you must agree to sign the informed consent document. If the study protocol changes or as new information becomes available, the research team is required to update you, after which you may also be asked to sign a new informed consent document.

The informed consent document is not a contract; you are free to leave the study if any new information leads you to want to do so. In fact, you are free to leave the study at any time, for any reason.

Clinical Trials and Insurance
When considering entering a clinical trial, it is important to understand which charges are covered by the clinical trial sponsor, which are covered by the patient, and which are covered by the insurance company. The study sponsor may cover some of the clinical trial costs, and most studies provide the drug or treatment free of charge.

Other costs may or may not be covered by health insurance plans, such as certain routine patient care costs, including:
• doctor’s visits
• hospitalizations
• laboratory tests
• procedures (e.g., bone marrow biopsies or lymph node biopsies)
• radiology tests
• routine medical care
• drugs that are not part of the study design

It is important to speak with the clinical trial sponsor and your insurance provider ahead of time to see what will be covered. Medicare beneficiaries may have their routine care costs covered if they are participating in federally funded clinical trials. Visit cms.gov for more information on this.

To determine the costs you will be responsible for, patients should:
• talk with your doctor, nurse, social worker, or the study contact person to find out whether the study drug is provided free of charge or offered at a lower cost from the drug company
• ask your health-care providers and insurance representative to tell you which expenses their insurance plan covers
• contact the drug manufacturer or advocacy groups for assistance if your insurance company will not pay for costs or denies their claims
• speak with organizations that may have information and suggestions to help appeal denied insurance claims, such as:
  - The National Coalition for Cancer Survivorship at 888-650-9127
  - The Patient Advocate Foundation at 800-532-5274
• ask your doctor or the research study contact to send information to your insurance company about the benefits of the study