

The LLS PedAL Master Trial:

A Clinical Trial for Acute Myeloid Leukemia Pediatric Patients

This study is designed to speed up the approval of acute myeloid leukemia (AML) drugs and bring safe and effective treatments to pediatric patients.

What is AML?

Acute myeloid leukemia (AML) is a type of cancer that begins in the bone marrow, the spongy tissue inside bones where blood cells are made. It is caused by damage to the DNA (genetic material) of a developing cell in the bone marrow. Once the marrow cell becomes a leukemia cell, it multiplies uncontrollably into billions of leukemia cells.

These cells, called “leukemic blasts”:

- Do not function normally
- Are able to grow and survive better than normal cells
- Block the production of normal blood cells, resulting in a lower number of healthy blood cells (red blood cells, white blood cells and platelets)

What are clinical trials?

Every new drug or treatment regimen goes through a series of studies called “clinical trials” before it becomes part of standard therapy. The following information will help you better understand clinical trials, how they are conducted and how they can work for you:

- Clinical trials are carefully designed and thoroughly reviewed by expert doctors and researchers to make them as safe and scientifically correct as possible.
- Patients in clinical trials are watched closely by their doctors, as well as by other members of their medical team, to ensure their safety.
- The trial can be either changed or stopped at any time if there is a problem. Patients who take part in a clinical trial also have the option to leave the trial at any time.
- Participation in a carefully conducted clinical trial may offer other therapy options.
- Patient participation in past clinical trials has resulted in the treatments we have today.

The Leukemia & Lymphoma Society (LLS) is leading the PedAL Master Trial to improve cancer treatment in children. This trial will be ongoing for many years and can be found at many centers. It involves:

- Academic research institutions
- Drug companies
- Biomarker and genetic analysis
- Regulators
- Cooperative Study Groups
- A clinical research organization to help LLS coordinate the study

The goal of LLS, through the PedAL trial, is to test more than one new treatment for AML in the same trial. The aim is to help speed up the time to get new approved treatments to more patients diagnosed with AML.

Your child has joined this study, now what?

1. You may have already provided consent for your child to receive cytogenetic screening and genomic testing (see reverse side).
2. Your child will get a bone marrow biopsy.
3. The bone marrow sample will be quickly sent for genomic testing.
4. The cytogenetic screening may take about three days. The genomic testing will take longer.
5. While you and your child are waiting for the results, your child may have to stay in the hospital.
6. Once your child’s unique leukemia biology results are received, the doctors will discuss with you and your child (if old enough) the treatment sub-studies that may be appropriate.
7. If your child’s results do not match any of the treatment sub-studies, the doctor will discuss other treatment options.
8. Each sub-study of the trial will focus on a different group of the AML patient population.
9. However, no matter what the treatment, there will be many tests, assessments and appointments.

What is cytogenetic screening/analysis?

This test uses a microscope to examine the chromosomes inside the cells. Chromosomes are the part of a cell that contain genetic information. Normal human cells contain 46 chromosomes (22 pairs, plus the two sex chromosomes, which are different in males and females). The chromosomes are a certain size, shape and structure. In some cases of AML, the chromosomes of the leukemia cells have abnormal changes. The results of this test help plan your child's treatment.

What is genomic testing?

Genomic testing is the process of analyzing cells or tissue to look for changes in genes that may be a sign of a disease such as cancer. Genomic testing may be done using tumor tissue to help diagnose cancer, plan treatment, or find out how well treatment is working.

How long does the study last?

The trial may last many years.

1. Your child's bone marrow sample will be sent for many tests, including cytogenetic and genomic testing, and you, your child (if old enough) and the doctor will receive the results.
2. Treatment will begin and will vary depending on which sub-study your child is placed in. Each sub-study has a different treatment "protocol," or plan, which includes different drugs, different dosages and different durations of treatment.
3. A follow-up period will occur after treatment is over.

How is the LLS PedAL Master Trial different from other pediatric clinical trials?

This clinical trial is designed to speed up the process of finding more and better treatments for AML. A typical clinical trial studies either a single drug or a combination of drugs and compares the outcomes with those of a

standard treatment. The PedAL trial is designed with several treatment options so that researchers can look at more than one drug at a time and see how well they work in treating AML.

This trial is only open to pediatric patients whose cancer has relapsed (recurred after treatment). Most pediatric AML clinical trials are for patients who are newly diagnosed.

The LLS PedAL Master Trial has the potential to stand as a model for future pediatric cancer clinical trials.

Are there resources that can provide parents, guardians and the patient with more information?

Anyone who wants more information about AML, this clinical trial, pediatric resources, caregiver information or any other topic can talk with the doctor and healthcare team. They can also call, email or chat with an LLS Information Specialist (see below).

How can an Information Specialist help?

LLS Information Specialists are highly trained oncology social workers, nurses and health educators who are available to speak with you one-on-one to advise and help you and your family through treatment, financial and social challenges, and give accurate, up-to-date disease, treatment and support information.

- Call: (800) 955-4572
- Email and Live Chat:
www.LLS.org/InformationSpecialists

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Information Specialist: **800.955.4572**

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.