Beat AML Master Trial Ahead of Plan

Last fall, The Leukemia & Lymphoma Society (LLS) launched the paradigm-shifting Beat AML Master Trial to bring precision medicine to acute myeloid leukemia (AML), one of the deadliest blood cancers. The past 40 years have brought remarkable breakthroughs in blood cancer treatments—but virtually none for AML. Researchers developed more than 100 experimental therapies, but, until recently, all failed clinical tests because responses varied so dramatically by type of AML.

Today, with new technologies and new insights, researchers believe the path forward is precision medicine: individualizing treatments based on...
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The Master Trial is running ahead of schedule as it reaches its first year.
- More than 140 patients are enrolled at seven treatment centers, with three additional centers to open by the end of this year.
- Seven studies are being conducted at each site, and plans are underway to add three additional studies.
- We are beginning to demonstrate that precision medicine is feasible for AML, even though the rapid progression of AML leaves little time for genetic screening before treatment. By pushing the boundaries of technology, we complete each patient’s screening and targeted treatment recommendation within just seven days.

The Master Trial’s impact will be felt well beyond AML, and even beyond blood cancer. The treatments that emerge could help patients with other diseases that share genetic mutations with AML.

The design of the Master Trial is setting a new standard for clinical trials, not least because of the unprecedented collaboration that LLS has brought about among patients, academic institutions, pharmaceutical companies, and regulators. All stakeholders share a common goal: improve patient outcomes and save lives.

Most importantly, we are bringing new hope to patients through precision medicine.

To learn more, please visit: lls.org/beat-aml.

LLS is the first nonprofit health organization to sponsor a cancer clinical trial. Bringing new hope to patients through precision medicine, the treatments that emerge from this AML Master Trial could help patients with diseases that share similar genetics to AML.

Supporter’s Corner

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money for LLS. A few years later, I wanted to do another marathon and again turned to TNT. After that I was hooked and continued with TNT for another ten plus years. I kept coming back because I saw the results of research in the lives of so many honorees whose lives were extended by changes in treatments.

As a twenty-five year survivor of CLL, I have been impacted personally by LLS research. Prior treatments were a result of LLS research and my current treatment plan was funded early on by LLS research funds. I continue to support LLS because I can see the action of LLS driven by its mission statement. LLS is truly making a difference in survivorship and patient and family quality of life. Its research and patient services keep me as a donor and supporter. I will continue to support LLS making someday today.

Lois Markovich
San Francisco, CA

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the patient’s type of AML. Instead of one treatment for all patients, we are now looking for many treatments that each target one type of AML.

LLS is simultaneously testing multiple therapies against multiple types of AML at multiple clinical sites, accelerating the testing and approval of promising therapies.

To support research in the area of greatest concern to you, contact us at 1-888-773-9958.

To speak to an Information Specialist call 800.955.4572 • To donate over the phone call 914.949.5213 • visit us at lls.org
Immunotherapy Approval: New Era in Cancer Treatment

Since August, the U.S. Food and Drug Administration (FDA) has approved two CAR (chimeric antigen receptor) T-cell immunotherapies, bringing an entirely new approach to treating cancer: supercharging a patient’s immune system to find and kill cancer cells.

The two new immunotherapies provide a life-saving new option for patients for whom previous treatments have failed. The Novartis CAR-T immunotherapy was approved for children and young adults with B-cell acute lymphoblastic leukemia (ALL) and the Kite Pharma CAR-T immunotherapy for several types of relapsed and refractory non-Hodgkin lymphoma.

Over the past 20 years, LLS has invested $40 million in CAR-T, helping to pioneer this life-saving technology. Through its Specialized Center of Research Program, LLS advanced Novartis’s therapy at the University of Pennsylvania. LLS funded the Kite, a Gilead Company, immunotherapy clinical trial through its Therapy Acceleration Program®, a strategic initiative through which LLS partners with biotechnology companies to accelerate promising therapies.

Children like Austin Schuetz (at left) are why immunotherapy is so important. At age five, Austin had battled leukemia for most of his young life. After chemotherapy and stem cell transplantation failed him, his parents enrolled him in a clinical trial to receive the new treatment. Today, at age 10, he is in remission.

According to LLS president and chief executive officer, Louis J. DeGennaro, PhD, “Immunotherapy is dramatically changing the way we approach blood cancer, and we are hopeful that this therapy will ultimately be applicable to patients with other types of cancers as well.”

To learn more, please visit: lls.org/car-t-cell-immunotherapy.

10-year-old Austin Schuetz is in remission after receiving a revolutionary new immunotherapy treatment in an LLS-funded clinical trial.
Four Therapies Receive FDA Approval and Bring New Hope to AML Patients

This past summer brought exciting headlines for AML patients: the FDA approved four new therapies. After few changes to the standard of care in the past 40 years, these approvals signal the beginning of more effective treatments.

LLS invests at the forefront of AML research—nearly $100 million in the past five years alone. These investments yield new therapies and new insights that lay the groundwork for the next generation of treatments.

The four new treatments offer new hope to patients with several types of AML.

Novartis’s midostaurin (Rydapt®) is for patients with a FLT3 mutation which accounts for approximately 30 percent of patients.

Celgene and Agios’s enasidenib (IDHIFA®) is for AML patients with an IDH2 mutation which includes about 12 percent of patients.

Jazz Pharmaceutical’s CPX-351 (Vyxeos™) is an innovative reformulation of two chemotherapies for patients with secondary AML, a high-risk subtype that occurs in 10-20 percent of AML patients and has a very poor prognosis.

Pfizer’s gemtuzumab ozogamicin (Mylotarg®) is for certain patients with a CD33 protein, including newly diagnosed adults and children aged 2 years and older who have relapsed or for whom initial treatment was ineffective.

These new treatments were made possible by forward-looking investors, like LLS and our supporters. Today, we are looking ahead to the next frontier: precise, life-saving therapies for every type of AML.