



LLS's Leadership Role in Acute Myeloid Leukemia

As the world's largest voluntary health agency dedicated to fighting blood cancers, The Leukemia & Lymphoma Society (LLS) is leading the offensive to dramatically improve outcomes for patients with acute myeloid leukemia (AML).

LLS began funding AML research at our inception 67 years ago, and about 26 percent of our annual research budget goes to AML research. In the past five years alone we have invested nearly \$100 million in AML research, with a focus on understanding the underlying causes of the disease to develop better therapies and save more lives.

LLS had the vision to make a “down payment” for patients with AML in 2009 by partnering with Celator Pharmaceuticals to advance CPX-351, an innovative formulation of two existing therapies, which performed well in a Phase 3 clinical trial for patients with secondary AML, a high-risk subset of the disease. This investment has the potential to pay off significantly as the first new treatment in the US in 40 years for patients diagnosed with AML.

Forging a Path for AML Patients

Through our Beat AML initiative, LLS is fostering collaboration among researchers at multiple institutions, regulators, pharmaceutical and biotechnology companies, primary healthcare physicians and patients, to develop effective, individualized therapies to treat patients with AML.

There are currently more than 100 therapeutic agents being studied for AML, which remains one of the most lethal blood cancers. Key areas of research are focused on targeting the different mechanisms that cause the disease, and among these are stem cell research, studying the cancer microenvironment, and harnessing the immune system.

- LLS currently supports several leading edge AML projects through our **Therapy Acceleration Program**:
 - **Celator**: LLS supported Phase 2 and 3 clinical trials of CPX-351, an innovative formulation of two existing chemotherapy drugs used to treat AML. CPX-351 performed well in the Phase III trial for patients with secondary AML, a high-risk subset of the disease.
 - **University of Florida**: LLS supported the Phase 1B/2 clinical trial of an investigational drug, OXi4503, for treatment of AML patients. OXi4503 is a vascular disrupting agent (VDA) for treatment of cancer patients under development by OXiGENE, Inc., a biopharmaceutical company.

- **Kiadis Pharma N.V.:** LLS is funding the Phase II development of Kiadis Pharma's lead product, ATIR101™, through an equity investment of approximately \$1 million. This ongoing Phase II trial is investigating the repeated dosing of ATIR101™ as an adjunctive treatment to a T-cell depleted haploidentical hematopoietic stem cell transplantation (HSCT) (donor cells from a half-matched related donor) in adult patients with acute myeloid leukemia (AML) or acute lymphoblastic leukemia (ALL).
- Further, LLS is currently supporting 66 AML projects through our **academic grants portfolio**, including:
 - **Irene Ghobrial, M.D., Dana-Farber Cancer Institute:** Specialized Center of Research grant focused on preventing the progression of precursor conditions such as myelodysplastic syndromes (MDS) and myeloproliferative neoplasms (MPN) to the more lethal acute myeloid leukemia (AML).
 - **Roland Walter, M.D., Ph.D., Fred Hutchinson Cancer Research Center:** Translational Research Program grant supports research to develop new highly active antibody-based treatments for acute leukemia. Dr. Roland's work is focused on engineering bispecific antibodies that engage immune cells to target tumor cells in acute leukemias.
 - **Stephen Gottschalk, M.D., Baylor College of Medicine:** Translational Research Program grant is supporting his team's development of an immunotherapy approach for AML with T cells that are genetically modified to maximize anti-tumor activity. The genetically modified T cells can be switched off if needed to minimize potential side effects.
 - **Matthew Shair, M.D., Harvard University:** Developing a small molecule drug that potently and selectively inhibits the proliferation of MLL-rearranged leukemias, aggressive forms of acute myeloid leukemia (AML) or acute lymphoblastic leukemia (ALL) with poor prognosis and few treatment options. Recently Merck paid \$20 million to buy the rights to continue the optimization of the compound for future clinical use.
 - LLS is also supporting a special one-year \$400,000 grant at **University of California, San Diego** that is advancing a novel technology to deliver RNAi therapeutics (RNA interference – inhibiting gene expression) to cancer cells to eradicate them.
 - LLS is conducting a research study to understand AML patient experiences and expectations, and preferences for the benefits and risks of treatment. The results of this “**patient preference study**” will be used to help ensure that both the FDA and drug manufacturers better reflect their preferences in the drug development process.