

Children's Hospital Los Angeles initiates clinical trial for treatment-resistant leukemia

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The Children's Center for Cancer and Blood Diseases (CCCBD) at Children's Hospital Los Angeles is one of the first sites in the world to offer a promising new investigational therapy to treat pediatric acute lymphoblastic leukemia (ALL).

The most common cancer diagnosed in children, ALL accounts for 25% of pediatric malignancy. Although the cure rate is high for pediatric ALL, the disease remains a leading cause of cancer related mortality in children.

This new trial was initiated in conjunction with Santa Monica-based biopharmaceutical company Kite Pharma, Inc. The study, referred to as ZUMA 4 (NCT02625480), tests a novel therapy called KTE-C19, which uses a patient's own T [cells](#) to target the antigen CD19, a protein expressed on the cell surface in most cases of childhood ALL.

Alan S. Wayne, MD, director of the CCCBD, is the lead principal investigator for this clinical trial that is now open to patients with ALL whose disease is resistant to, or has relapsed following, standard chemotherapy or stem cell transplant.

"This approach has ushered in a whole new era of cancer immunotherapy," says Wayne, who is also associate director for Pediatric Oncology at the USC Norris Comprehensive Cancer Center and Professor of Pediatrics at the Keck School of Medicine, University of Southern California.

In this trial, T cells - a primary type of immune cell - are genetically engineered to recognize the CD19 protein on the surface of leukemia cells. In this approach, the patient's own T cells are collected and modified in the laboratory to express what is called a chimeric antigen receptor (CAR). The T cells expressing the CAR are then returned to the patient where they can attack and eliminate leukemia cells.

Wayne previously led the development of the predecessor trial that treated 21 children at the Pediatric Oncology Branch of the National Cancer Institute at the National Institutes of Health. The results of that study, first published online in the medical journal *Lancet* in 2014, showed unprecedented complete response rates of 70 percent in [children](#) with relapsed or chemotherapy refractory ALL. An accompanying editorial stated: "This approach is without question the most significant therapeutic advance in ALL for a generation, and might represent the beginning of a new era of engineered T cells for cancer therapy".

The CCCBD is the largest pediatric hematology, oncology and blood and marrow transplant program in the western United States. In 2015, The U.S. News & World Report ranked the CCCBD as the top pediatric hematology/oncology program in California and #8 in the nation.

This new study is part of the CCCBD's growing Cellular Therapeutics Program designed to propel the next generation of innovation in childhood [cancer](#).

Provided by Children's Hospital Los Angeles

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