

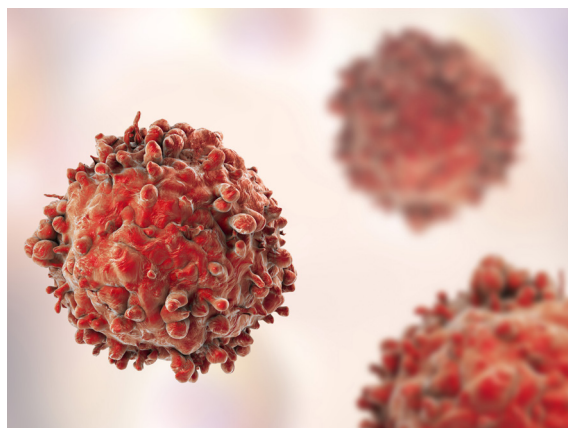


# INSIDE PEDIATRICS

## GROUNDBREAKING LEUKEMIA TREATMENT NOW AVAILABLE AT CHILDREN'S

Children's of Alabama is now one of about 35 healthcare institutions in the U.S., and the only pediatric provider in Alabama, chosen to offer a new FDA-approved immunotherapy called chimeric antigen receptor (CAR) T-cell therapy.

Children's offers the treatment to children and young adults with pre-B cell pediatric acute lymphoblastic leukemia (ALL). ALL is a cancer of the bone marrow and the most common form of pediatric leukemia. About 3,000 children and adolescents are diagnosed with ALL in the U.S. each year. CAR T-cell therapy is designed for ALL that has returned after treatment or that is unresponsive to standard chemotherapy treatment. Typically, about 90 percent of ALL patients are cured with intensive chemotherapy treatment. The remaining patients don't respond to chemotherapy or relapse and have a poor prognosis.



The CAR T-cell therapy takes a patient's own immune cells, white blood cells that normally help fight infection, and programs the cells to recognize and destroy the patient's leukemia cells.

"CAR T-cell therapy is truly a breakthrough therapy in cancer treatment" said Matthew Kutny, M.D., director of the Leukemia, Lymphoma and Histiocytosis Program at the University of Alabama at Birmingham (UAB) Division of Pediatric Hematology-Oncology and The Alabama Center for Childhood Cancer and Blood Disorders at Children's. "This is the first FDA-approved treatment that involves engineering a patient's own immune cells in a laboratory to turn them into cancer fighting cells."

The FDA approved the CAR T-cell therapy on Aug. 30, following multicenter clinical trials at several sites around the country that demonstrated an 83 percent rate of remission in these very high-risk patients, and these remissions are long lasting in approximately 50 percent of patients.

"We have made remarkable progress in our fight against childhood cancer," Dr. Kutny said. "Today approximately 84 percent of children diagnosed with cancer are alive five years from their diagnosis but we know that many of our patients also develop long-term complications from their chemotherapy like heart disease or kidney problems. We want to cure 100 percent of children and do so with as few side effects as possible so that these children live long, healthy lives. Our center currently has several studies of immunotherapies (cancer treatments using antibodies and immune cells) that will help determine if these can provide safer and more effective treatments compared to chemotherapy."

One of the complications of CAR T-cell therapy is that patients can develop fevers and other ill symptoms as the CAR T-cells fight the leukemia like an infection. Kutny said investigators at Children's and UAB are working to create a new strategy in CAR T-cells that can help better control this immune response or shut it off once it is no longer needed. The FDA is requiring that all treatment sites providing CAR T-cell therapy have certification and training to handle potential risks and complications involved with this type of treatment like high fevers, low blood pressure and difficulty breathing that may require care in the intensive care unit. Children's is one of the select sites nationwide that has the required expertise and training to become a CAR T-cell treatment site.

More than 190 Alabama children are diagnosed with a form of childhood cancer or a blood disorder and 90 percent of those children are treated at Children's. Worldwide a child is diagnosed with cancer about every two minutes. Over 500 children undergo cancer treatments each year in Alabama.

"Pediatric leukemia is the first type of cancer with a FDA-approved indication for CAR T-cell therapy, but even more recently this type of treatment was also approved for adults with certain types of lymphoma," Kutny said. "In the near future we will see many others types of cancer treated with this type of cellular immunotherapy."