

THE IMMUNOTHERAPY PROGRAM AT CHILDREN'S HOSPITAL OF PHILADELPHIA

Immunotherapy refers to treatments that harness a patient's own immune system to help fight diseases, including cancer. Some therapies boost the power of the body's immune system so it works harder to fight off illness. Others involve introducing a man-made substance into the body that's designed to target and kill cancer cells. There are also approaches that reprogram a patient's own T-cells to recognize and attach to a protein that is found only on the surface of B cells.

Immunotherapy is widely recognized as a breakthrough in the treatment of cancer. While chemotherapy has been used successfully for decades to treat many cancer patients, there are some patients for whom chemotherapy does not cure their cancer. And for these patients, when cancer returns, it can be very difficult to treat again.

At CHOP, our goal is to bring the most effective treatments to the most patients, as quickly as possible.

Our Cancer Immunotherapy Program was designated a Frontier Program by Children's Hospital of Philadelphia in 2015. Frontier Programs are unique, cutting-edge programs that will forge important new discoveries, deliver novel therapies, and help even more children thrive.

To that end, our team:

- Began using immunotherapy known as 'CAR' T-cell therapy as an investigational treatment option for children with relapsed or refractory acute lymphoblastic leukemia (ALL) in 2012.
- Has treated over 440 CAR T cell patients to date since then.
- Has led clinical trials into the first form of CAR T-cell therapy to be approved by the U.S. Food and Drug Administration (FDA) for the treatment of relapsed and refractory pediatric acute lymphoblastic leukemia (ALL). The therapy is the first personalized cellular therapy for the treatment of cancer to receive FDA approval.
- Was the first to use investigational CAR T-cell therapy in a child with ALL, who remains cancer-free.
- Has the largest cohort of pediatric patients treated with CAR T cells targeting CD19 and the longest follow-up.
- Identified and applied a treatment to decrease the severity of potentially fatal toxic side effects of CAR T-cell therapy. This approach is now used worldwide for children and adults treated with CAR T-cells and other new immunotherapies.
- Continues to research the use of CAR T-cell therapy in the treatment of several other forms of pediatric cancer, with the hopes of expanding its use to fight other forms of leukemia and solid tumors.