

GD2-ZAP70 CAR T-Cell Clinical Trial for High-Risk Neuroblastoma

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Background

Neuroblastoma is the most common solid tumor occurring in young children. Even with intense chemotherapy, radiation, surgery, stem-cell transplant, and the current best immunotherapy, children diagnosed with this disease have only a 50–60% chance of long-term survival. After all that treatment, if a child relapses, most will not survive. More effective and less toxic therapies are urgently needed.

CAR T-cell therapy is a type of immunotherapy that turns a patient's own immune cells into a "living drug" that eradicates cancer. CAR T-cell therapy has already revolutionized treatment for many adult cancers as well as pediatric leukemia and is now beginning to help some children with neuroblastoma. In fact, the largest single neuroblastoma treatment advance in the past two decades was the discovery of an antibody which recognizes a specific sugar found on the surface of the tumor cells called GD2. This anti-GD2 antibody is the only drug FDA-approved specifically for the treatment of any pediatric solid tumor compared to more than twenty for adult cancers. While some patients with very small amounts of disease have responded well, most patients do not because current GD2 CAR T-cells get "exhausted" quickly and stop working. A stronger, longer-lasting version of this therapy is needed to help the majority of children diagnosed with neuroblastoma.

New GD2-ZAP70 CAR-T Cell Clinical Trial for High-Risk Neuroblastoma

Robbie Majzner, MD and his team at Dana-Farber Cancer Institute have invented a completely new kind of CAR T-cell that solves this problem of T-cell exhaustion. Their new GD2-ZAP70 CAR replaces the traditional CAR construction with a piece of a natural immune protein called ZAP70. In head-to-head laboratory and animal studies, this simple change produced dramatic improvements: stronger and more sustained elimination of neuroblastoma tumors in mouse models.

With your support, a Phase 1 clinical trial will open in late 2026 for children and young adults whose high-risk neuroblastoma has come back or did not respond to standard therapy. In this trial, we will test the safety of this approach, find the right dose, as well as measure how well the new CAR T cells grow in the body, how long they last, and whether they can shrink or eliminate neuroblastoma tumors.

Importance of Philanthropy

Philanthropy is critical towards launching clinical trials for children diagnosed with cancer. Pharmaceutical companies focus on more common adult cancers, and given the rarity of pediatric cancers including neuroblastoma, donor support is the only way to bring groundbreaking academic discoveries like GD2-ZAP70 CARs to children in the clinic. Dana-Farber has one of the world's top pediatric CAR T-cell programs, a state-of-the-art cell manufacturing facility, and a long track record of safely bringing new therapies from the lab to the bedside. With your support, we are poised to move quickly. The manufacturing process is underway, and the FDA submission is on track for the second half of 2026. Your gift will accelerate this trial, bringing a potentially transformative new therapy to children who do not have other options. We remain deeply grateful for your partnership. [Thank you.](#)