

# Results of glioblastoma clinical trial show safety and clinical benefit of CAR T cell therapy

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Glioblastoma is the most common brain tumor in humans and also one of the most difficult cancers to treat; patients with this type of cancer only survive about one year from time of diagnosis. Researchers at Baylor College of Medicine, Texas Children's Cancer Center, and the Center for Cell and Gene Therapy at Baylor, Texas Children's Hospital and Houston Methodist are investigating a new treatment option using modified T cells with anti-tumor properties with the goal of improving outcomes for patients with glioblastoma.

Their research focuses on engineered T cells that target the protein HER 2 expressed in low levels in [glioblastoma cells](#). Results of a Phase 1 study published in the current issue of *JAMA Oncology* established the safety of these HER 2-specific, chimeric antigen receptor modified T cells (CAR T cells) when infused in to patients in increasing doses and, importantly, results also showed a clinical benefit to patients.

"Our inability to effectively treat glioblastoma has been one of the failures of oncology," said Dr. Nabil Ahmed, associate professor of pediatrics at Baylor, Texas Children's Cancer Center and the Center for Cell and Gene Therapy and first author of the paper. "Glioblastoma is resistant to standard therapy, and it is difficult to remove all of the [tumor cells](#) through surgery without damaging the brain, so there is an urgent need for new and better treatment. Our work has focused on immune therapy, because it is very targeted and uses tumor-killing mechanisms that the cancers have not shown resistance to in the lab."

CAR T cells are T cells – a type of [immune cells](#) involved in the defense against tumors – that have been programmed to recognize and kill tumor cells carrying one specific antigen, in this case HER2, on the surface of [cancer cells](#) through an artificial

molecule, the CAR, expressed on their surface.

The study included 17 pediatric and adult patients with HER 2-positive glioblastoma who received up to five escalating doses of the engineered T cells through intravenous infusions. Establishing the safety of the treatment is important, as other immunotherapy treatment approaches for solid tumors have resulted in significant side effects and toxicities for patients, Ahmed said.

"First and foremost, the cells were safe. We did not see any life threatening side effects. Along with this we also saw measurable tumor responses," Ahmed said.

Median survival of patients who participated in the trial was 11.1 months post T cell infusion and 24.5 months from diagnosis. Three [patients](#) in the trial experienced no disease progression after more than two years of follow up.

With their promising results, Ahmed and his research colleagues, including Dr. Stephen Gottschalk, professor of pediatrics at Baylor, Texas Children's Cancer Center and the Center for Cell and Gene Therapy and senior author of the paper, turn their focus to the next steps in the research.

"In this phase 1 clinical trial we tested a particular modification that renders these cells specific for HER 2 and while the results have been encouraging, we are very interested to further engineer these cells, for example by making the T cells more effective after the infusion and by targeting not only HER2 but other molecules that are expressed on the cell surface of brain tumors," Gottschalk said.

The CAR T [cells](#) are produced in the cell manufacturing facility of the Center for Cell and

Gene Therapy. Baylor is one of the few academic institutions that has such a facility, Gottschalk noted.

**More information:** Nabil Ahmed et al.  
HER2-Specific Chimeric Antigen  
Receptor–Modified Virus-Specific T Cells for  
Progressive Glioblastoma, *JAMA Oncology* (2017).  
[DOI: 10.1001/jamaoncol.2017.0184](https://doi.org/10.1001/jamaoncol.2017.0184)

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