Regression of Glioblastoma after Chimeric Antigen Receptor T-Cell Therapy.

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**Public Summary:**
A patient with recurrent multifocal glioblastoma received chimeric antigen receptor (CAR)-engineered T cells targeting the tumor-associated antigen interleukin-13 receptor alpha 2 (IL13Ralpha2). Multiple infusions of CAR T cells were administered over 220 days through two intracranial delivery routes - infusions into the resected tumor cavity followed by infusions into the ventricular system. Intracranial infusions of IL13Ralpha2-targeted CAR T cells were not associated with any toxic effects of grade 3 or higher. After CAR T-cell treatment, regression of all intracranial and spinal tumors was observed, along with corresponding increases in levels of cytokines and immune cells in the cerebrospinal fluid. This clinical response continued for 7.5 months after the initiation of CAR T-cell therapy.

**Scientific Abstract:**
A patient with recurrent multifocal glioblastoma received chimeric antigen receptor (CAR)-engineered T cells targeting the tumor-associated antigen interleukin-13 receptor alpha 2 (IL13Ralpha2). Multiple infusions of CAR T cells were administered over 220 days through two intracranial delivery routes - infusions into the resected tumor cavity followed by infusions into the ventricular system. Intracranial infusions of IL13Ralpha2-targeted CAR T cells were not associated with any toxic effects of grade 3 or higher. After CAR T-cell treatment, regression of all intracranial and spinal tumors was observed, along with corresponding increases in levels of cytokines and immune cells in the cerebrospinal fluid. This clinical response continued for 7.5 months after the initiation of CAR T-cell therapy. (Funded by Gateway for Cancer Research and others; ClinicalTrials.gov number, NCT02208362.).