

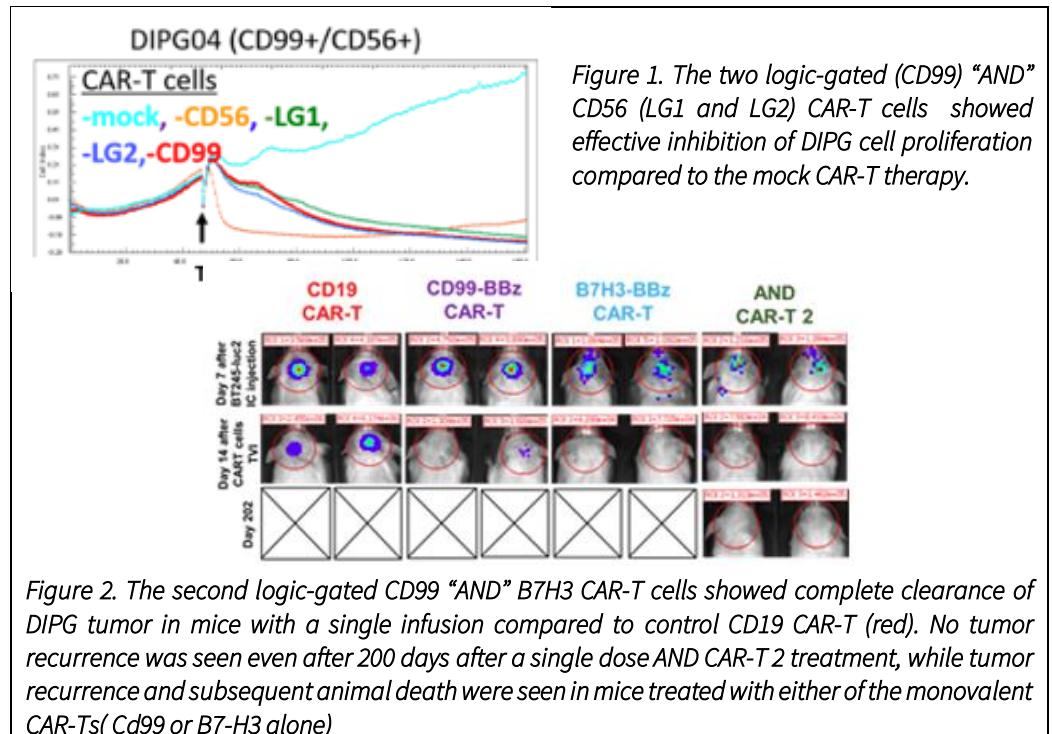
# Logic Gated CAR-T Therapy for Pediatric Brain Tumors

## Diffuse Intrinsic Pontine Glioma

DIPG (Diffuse Intrinsic Pontine Glioma) is a highly aggressive brain tumor in children, primarily located in the pons region. It's inoperable due to the tumor's location, and chemotherapy agents have the inability to cross the blood-brain barrier. Radiation offers only temporary relief, and the 5-year survival rate has remained at 0% for decades. CAR-T cell therapy, successful in blood cancers, faces challenges in treating solid tumors due to the absence of unique "tumor-only" antigens that are not shared with normal cells.

## Logic Gated CAR-T

A University of Colorado research group led by Drs. Venkataraman and Kohler have developed and assessed the functionality of innovative 'logic-gated' AND CAR T-cells. They are designed to target two distinct antigens highly co-prevalent on DIPG cells and not co-expressed in normal cells. There are two specific logic-gated CAR-T versions: one targeting CD99 and CD56 (also effective in AML) and another targeting CD99 and B7H3 (also effective in Ewing sarcoma and Ependymoma). These novel CAR T-cells mark the pioneering approach to targeting DIPG-specific antigens and represent the first application of logic-gated CAR-T cell therapy for DIPG.



### Advantages:

- Novelty is in identifying the two antigens to co-target to combat DIPG
- Efficiently targets tumor cells regardless of antigen expression levels, preventing escape
- Enhances safety by dual targeting of tumor antigens using logic-gated AND technology

### Products

CD99 and CD56 CAR T-cell Therapy  
CD99 & B7H3 CAR T-cell Therapy

### Indication

Diffuse Intrinsic Pontine Glioma (DIPG)  
Ependymoma  
glioblastoma  
Ewing Sarcoma  
Acute Myeloid Leukemia (AML)

### Value Propositions

- ▶ 0% DIPG 5-year survival rate
- ▶ Current standard of care is limited to radiation
- ▶ Logic gated CAR-T therapy are effective at killing tumor cells and limiting off-target toxicities

### Market

- ▶ In US, up to 20% of all pediatric brain tumors are DIPG: 300 diagnosis/year
- ▶ Ependymoma: 143 million (2022), 4.8% CAGR (2022-2030)
- ▶ High-grade glioma: \$1.1 B (2017, 7MM), 3.8 % CAGR (2020-2030)
- ▶ Ewing Sarcoma: 9,457 cases (2023, Global), \$245 M (2022, Global), 5.7% CAGR (2022-2032)
- ▶ AML: \$1.4 B (2010, 8MM), 13.6% CAGR (2019-2029)

### Intellectual Property

- ▶ PCT stage: Application Filed US 63/489,718

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