Innovations
UNIVERSITY OF COLORADO
ANSCHUTZ MEDICAL CAMPUS

CAR-T Targets Novel CD99 in Pediatric Brain Tumor

Product

CD99 CAR T-Cell therapy

Indication

Diffuse Intrinsic Pontine Glioma
Ewing Sarcoma

Value Propositions

- 0% DIPG 5-year survival rate
- Current standard of care is limited to radiation
- CD99 CAR T cells are effective at killing tumor and are not toxic

Market

- In US up to 20% of all pediatric brain tumors are DIPG: 300 diagnosis/year
- 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)

Intellectual Property

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

Challenges in DIPG Treatment

The field of Diffuse Intrinsic Pontine Glioma (DIPG) tumors faces a formidable challenge in the realm of pediatric oncology. These tumors, located in the brainstem, are inoperable due to their diffuse and infiltrative nature, rendering surgical removal impossible. Furthermore, the blood-brain barrier poses a significant obstacle, making it extremely challenging for traditional chemotherapeutics to reach the tumor site effectively. Currently, the primary treatment option for DIPG is radiation therapy, which provides only temporary relief from clinical symptoms but rarely results in long-term survival. The prognosis for DIPG remains overwhelmingly bleak, with a median survival rate of less than a year from diagnosis. The urgent need for more effective and innovative therapies in this field underscores the complexity and gravity of the problem.

CD99 Targeting CAR-T

A University of Colorado research group led by Drs. Venkataraman and Vibhakar has developed a groundbreaking anti-CD99 scFv (10D1) and CAR-T cell therapy based of this scFv that is specific to humans, avoiding cross-reactivity with murine CD99, unlike current therapies. These novel CD99 CAR-T cells express high levels of CD99 receptors and have demonstrated remarkable efficacy against the aggressive childhood brain tumor, DIPG, in murine models. Importantly, they do not exhibit toxicity or cross-reactivity with murine skin. Engineered CD99 CAR-Ts cured DIPG in murine models, and was found safe to normal brain cells. In addition, low-dose radiation enhances therapy by inducing CD99 antigens on tumor cells, increasing efficacy and potentially allowing for lower CAR T cell doses, reducing off-target side effects.

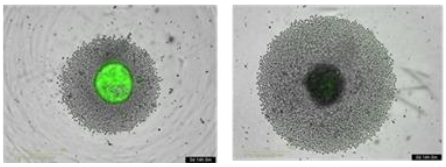

Figure 1. The CD99 CAR-T cells completely killed the DIPG cells (right), while CD19 control CAR-T did not affect DIPG cell viability. DIPG cells (green), dead cells (black), and CAR-T cells (grey).

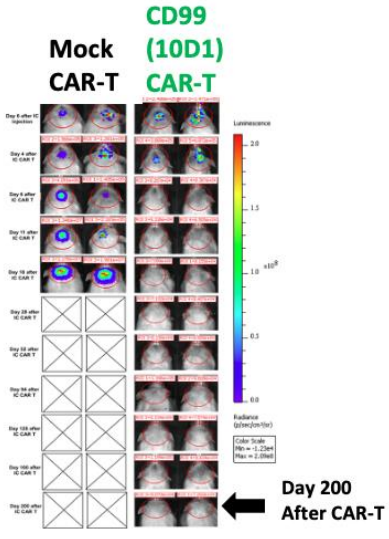

Figure 2. Complete clearance of DIPG tumor with a single dose of CD99 CAR-T cell treatment (right) delivered intrathecally compared to the Mock control CAR-T (left).

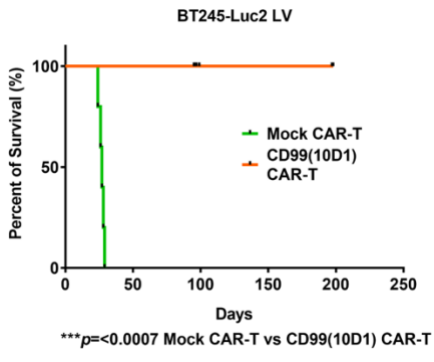

Figure 3. 100% survival rate at day 50 with CD99 CAR-T vs. 0% survival rate at ay 50 with the Mock CAR-T.

Contact

Doreen Molk
Doreen.molk@cuanschutz.edu

Ref# CU6129H

303-724-0220
cuanschutz.edu/cu-innovations

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