Docket #: S18-273

CD93-targeting CAR-T to treat AML

Hematological cancers like non-Hodgkin's lymphoma have seen a revolution in care due to the advances of targeted cell therapies like CAR-T. However, not all blood cancers have enjoyed the same benefit, hampered by the lack of actionable and specific blood cell targets. Researchers at Stanford have identified and patented a novel target that is selectively expressed on cancer-causing cells in patients with acute myeloid leukemia (AML), and raised a humanized antigen against the target to create a CAR-T therapy with therapeutic activity in vivo.

Clinical development in AML has more recently shifted towards targeted therapies defined by genetic populations- a strategy shift that has not yet trickled down to cell therapies, given the multiple antigens expressed on leukemia cells and the significant variety in antigen expression profiles between patients. However, the CD93 target identified by the inventors is constrained to leukemia progenitor populations, suggesting an ideal expression profile for an anti-cancer therapy.

Indication: Acute myeloid leukemia (AML)

Drug format: Cell therapy

Drug class: First in class

Target: CD93

Stage of Development:

- *In vitro:* CD93-targeting CAR-T cells produced cytokines IL2 and IFN in response to exposure to two separate AML cell lines, along with killing of the tumor cell lines.
- *In vivo:* In a mouse model of AML, animals treated with CD93-targeting CAR-T cells via tail vein injection saw clearance of leukemia cells within seven days, while untreated mice saw engraftment maintained. Survival was significantly increased in treated animals, with T-cells persisting for at least 21 days in vivo.

Applications

- Targeted AML therapy
- Novel CAR-T construct
- Unique oncology target profile
- Novel humanized chimeric antibody

Advantages

- One of few cell therapy candidates in AML
- Targeted therapy in a heterogeneous disease
- Applications in other myeloid diseases

Publications

- Richards, R. M., Zhao, F., Freitas, K. A., Parker, K. R., Xu, P., Fan, A., ... & Mackall, C. L. (2021). NOT-gated CD93 CAR T cells effectively target AML with minimized endothelial cross-reactivity. *Blood Cancer Discovery, 2*(6), 648-665. https://doi.org/10.1158/2643-3230.BCD-20-0208
- Majeti, R., MacKall, C., Liu, J., Majzner, R., Richards, R., & Hong, W. J. (2025). *U.S. Patent No. 12,371,469*. Washington, DC: U.S. Patent and Trademark Office.

Patents

- Published Application: WO2020180706
- Published Application: 20220133794
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