

# EXUMA Biotech Presents Early Clinical and Translational Findings from Solid Tumor Cell Therapy and In Vivo CAR Engineering Research at the AACR Annual Meeting 2023



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**EXUMA Biotech Corp.** →  
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WEST PALM BEACH, Fla., April 17, 2023 /PRNewswire/ -- EXUMA Biotech, Corp., a clinical-stage biotechnology company discovering and developing cell and gene immunotherapies for solid and hematological tumors, presented a poster entitled "*In vivo* delivery of CD3-directed CD19-CAR lentivectors leads to the generation of CAR T and NK-like (CAR-TaNK) cells capable of complete ablation of B cells in the blood, bone marrow, and tissue of NSG-SGM3 CD34+ humanized mice" and gave an oral presentation in the "25th Anniversary of Trastuzumab: Impact and Future Directions" major symposium at the American Association of Cancer Research Annual Meeting 2023 held in Orlando.

EXUMA's presentation, "Can cellular therapy provide another arrow in the quiver for HER2 positive malignancies?" reviewed the potential for novel modalities to continue targeting HER2 in late-stage HER2-positive cancers and provided translational and clinical insights from a novel HER2 targeted CAR-T product (CCT303-406) developed by the company in addition to next-generation technologies. Similar to many solid tumor targets, early clinical research with CAR-T therapies targeting HER2 were challenged by on-target, off-tumor toxicity. EXUMA

developed CCT303-406 to provide Tumor Microenvironment Restricted (TMR) CAR binding based upon characteristics unique to the tumor microenvironment. Updates from an investigator-initiated, dose escalation study in patients with advanced relapsed/refractory HER2+ solid tumors reported no dose-limiting toxicities to date attributable to CCT303-406 through 9 patients across the planned dose cohorts. Encouraging, early evidence of clinical activity correlated with subjects with cell expansion in the periphery at the highest planned dose cohort ( $1 \times 10^7$  cells/kg), with post-treatment tumor biopsy data suggesting enrichment of CCT303-406 CAR T cells within the tumor. Based upon the clinical profile to date, further dose escalation, enrichment within select HER2-overexpressing malignancies, and backfill of the  $1 \times 10^7$  cells/kg cohort is planned.

Dr. Gregory Frost, Chairman & CEO, remarked that "The safety profile to date, the evidence of pharmacokinetic amplification of CCT303-406 in patients receiving higher doses of therapy, and preliminary evidence of clinical activity, see us cautiously optimistic that our preclinical findings will continue to translate in the clinic. Investigation of higher doses in defined HER2+ malignancies may be warranted to establish CCT303-406's complete safety profile and potential efficacy in these patient populations with significant unmet clinical needs."

Also today, Dr. Sidharth Kerkar, VP, Research & Development, presented preclinical data highlighting the ability of the Company's next-generation CAR platform (GCAR) to generate CAR cells *in vivo* and eliminate CD19 B cells in a dose-dependent manner following direct administration of a CD3-directed lentivector encoding a CD19 CAR and EXUMA's proprietary FITNESS DRIVER.

Dr. Kerkar commented "We're exceptionally pleased with the progress EXUMA is making with its *in vivo* CAR therapy program, and excited that our FITNESS DRIVER generates the unique CAR-TaNK effector cell phenotype with both T and NK features when incorporated into GCAR."

About CCT303-406

CCT303-406 is EXUMA Biotech's tumor microenvironment restricted (TMR) autologous CAR-T product candidates targeting HER2, which is currently part of an investigator-initiated clinical trial in patients with metastatic HER2+ solid tumors. HER2 overexpression is a hallmark of several tumors, including those originating from breast, stomach, bladder, and colon. A

significant proportion of patients relapse or become unresponsive to antibody-based products targeting HER2 in early lines of treatment, yet still retain overexpression of HER2. CCT303-406 may be a promising option for this patient population providing T cell-mediated antitumor activity via targeting of HER2. Differentiating itself from other HER2 CAR-T therapies, CCT303-406 incorporates EXUMA's TMR safety technology, which helps restrict CAR-T activity to the tumor microenvironment potentially reducing the risk of on-target, off-tumor cytotoxicity.

## About the FITNESS DRIVER

EXUMA Biotech's FITNESS DRIVER is a proprietary, synthetic, intracellular, membrane-bound protein composed of two homodimers identified from an unbiased, comprehensive, *in vitro* and *in vivo* screen of thousands of signaling pairs for the ability to drive optimal *in vivo* proliferation, persistence, and cytotoxicity of CAR+ cells in the absence of lymphodepletion. The FITNESS DRIVER is encoded within the Company's next-generation CAR therapy lentivectors (LVs) that power the rPOC and GCAR platforms, both of which are engineered to generate CAR-T cell therapy in the patient, preserving T cell stemness and eliminating lengthy and costly *ex vivo* manufacturing steps. Moreover, the CD3-positive lymphocytes transduced with the FITNESS DRIVER take on a unique effector cell phenotype with features of both T and NK (TaNK) cells (CD3+, NKG2D+, CD8+, CD56+). EXUMA is the only company to have conducted a comprehensive screen of candidate pairs of signaling proteins to optimize the biology of CAR-T cells for safety, efficacy, and potentially eliminating the need for lymphodepleting chemotherapy prior to cell therapy, all of which are designed to improve and expand the setting of CAR-T cell therapy beyond the transplant ward.

## About GCAR

GCAR is EXUMA Biotech's next-generation, *in vivo* CAR engineering, LV platform. GCAR LVs have the potential to be directly administered to patients and target T cells through a CD3-directed element on the LV surface. Once inside the patient's T cells, the LV payload encoding a CAR and the FITNESS DRIVER produce T cells with enhanced proliferation, persistence, and greater cytotoxicity compared to traditional CAR-T cells. The modular nature of the GCAR platform may allow for other CARs to function with the FITNESS DRIVER, thereby enabling a robust, off-the-shelf, CAR therapy platform without the need for preparative chemotherapy and *ex vivo* cell processing.

## About EXUMA Biotech

EXUMA Biotech is a clinical-stage biotechnology company pioneering the discovery and development of novel cell and gene platform immunotherapies for the treatment of solid and hematological tumors. The company is leveraging a global R&D footprint to advance product candidates designed to have reduced cost and complexity with increased safety, efficacy, and scalability compared to existing cell therapies. For more information, visit [exumabio.com](http://exumabio.com) and connect with us on LinkedIn and Twitter (@ExumaBiotech).

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