



## **Clinical and Pre-Clinical Data on Mustang Bio's MB-101 (IL13R $\alpha$ 2-specific CAR T cells) for the Treatment of Glioblastoma (GBM) Presented at the 21<sup>st</sup> Annual Meeting and Education Day of the Society for Neuro-Oncology**

*Phase 1 data suggests MB-101 is safe and well-tolerated, capable of eliciting potent anti-tumor response*

**New York, NY – November 21, 2016** – Mustang Bio, Inc. (“Mustang”), a Fortress Biotech (NASDAQ: FBIO) Company, today announced that Phase 1 clinical data and pre-clinical data on its MB-101 (IL13R $\alpha$ 2-specific Chimeric Antigen Receptor–engineered CAR T cells (CAR T cells)) product candidate in development for the treatment of glioblastoma were presented by investigators from the City of Hope (“COH”) in oral sessions at the 21<sup>st</sup> Annual Meeting and Education Day of the Society for Neuro-Oncology (“SNO”) in Scottsdale, AZ.

Dr. Lindsay A. Rosenwald, Fortress Biotech’s Chairman, President and Chief Executive Officer commented, “CAR T cell therapy has shown promise in treating certain forms of hematological cancers. However, translating that activity into solid tumors has been challenging to date. The clinical data presented at SNO by the investigators from COH suggest MB-101 is safe and well-tolerated, and capable of eliciting a potent anti-tumor response in patients with glioblastoma (GBM), a disease that is almost universally fatal. We believe this is the first evidence of activity of CAR T cells in the treatment of GBM. We are very encouraged by the early evidence of anti-tumor activity with MB-101 with five of seven post-surgical resection patients showing stable disease for >8 weeks and, in particular, one patient showing complete response for 7.5 months. Interestingly, this patient was the only patient of the seven to receive, on a compassionate use basis, the dual delivery of CAR T cells by both intracavitary and via intraventricular administration, which provides systemic CNS delivery of the CAR T cells. We look forward to continuing to advance MB-101 in Phase 1 studies and further exploring the dual delivery approach beyond this single patient experience.”

The following summarizes the oral presentations on November 18, 2016 at SNO:

### **[Phase I Study of Chimeric Antigen Receptor–Engineered T cells Targeting IL13R \$\alpha\$ 2 for the Treatment of Glioblastoma](#)**

Presenter: Christine E. Brown, PhD, Heritage Provider Network Professor of Immunotherapy, Associate Director, T Cell Therapeutics Research Laboratory, City of Hope National Medical Center/Beckman Research Institute

The Phase I study presented showed early clinical data evaluating IL13R $\alpha$ 2-targeted CAR T cell therapy for the treatment of glioblastoma. On this study, patients are treated on a four-week therapeutic regimen consisting of three weekly intracranial infusions of IL13R $\alpha$ 2-specific CAR T cells followed by one rest week for toxicity and disease assessment. To date, seven patients have been treated with local intracavitary delivery of the CAR T cells following surgical resection.

Some highlights from the presentation included:

- The treatment was well-tolerated in all patients treated – with No DLTs or therapy-related SAEs
- No grade 3 or higher toxicities attributed to the therapy were observed
- No CRS or Neurotoxicity was observed
- Only grade  $\leq 2$  fevers, headaches, myalgia, chills
- Best Response: 2 PD, 4 SD for >8-weeks, 1 SD  $\rightarrow$  CR following intraventricular CAR T therapy for 7.5 months

## [Development of murine IL13R \$\alpha\$ 2-targeted CAR T cells \(mIL13BB \$\zeta\$ \) for assessment of CAR T cell therapy in syngeneic glioma models](#)

Presenter: Darya Alizadeh, PhD, City of Hope National Medical Center/Beckman Research Institute

The pre-clinical research program presented discussed a murine IL13R $\alpha$ 2-targeted CAR T cell platform that was developed to evaluate parameters that impact the efficacy of CAR T cell therapy. Overall, the development of mIL13BB $\zeta$  CAR T cells and its applications will allow researchers to assess factors that may impact the efficacy of CAR T cells and provide invaluable information critical for combination therapies and clinical trial design. These studies may also provide important insights for improving therapeutic outcomes for patients with glioblastoma.

### **About Glioblastoma multiforme (GBM)**

Glioblastomas (GBM) are tumors that arise from astrocytes cells that make up the supportive tissue of the brain. These tumors are usually highly malignant (cancerous) because the cells reproduce quickly and they are supported by a large network of blood vessels. GBM is the most common brain and central nervous system (CNS) malignancy, accounting for 15.1 percent of all primary brain tumors and 55.1 percent of all gliomas. There will be an estimated 12,120 new glioblastoma cases in the U.S. in 2016. Malignant brain tumors are the most common cause of cancer-related deaths in adolescents and young adults aged 15-39, and the most common cancer occurring among 15-19 year olds in the U.S. (Brain Tumor Statistics. American Brain Tumor Association. December 2015). While GBM is a rare disease (2-3 cases per 100,000 person life years in the U.S. and EU), it is quite lethal with five-year survival rates historically less than 10 percent. Chemotherapy with temozolomide and radiation are shown to extend mean survival from approximately 12 to 15 months, while surgery remains the standard of care. GBM remains difficult to treat due to the inherent resistance of the tumor to conventional therapies. Treatment is further complicated by the susceptibility of the brain to damage, difficulty of the brain to repair itself and limitation to drugs crossing the blood brain barrier. Immunotherapy approaches targeting brain tumors offer promise over conventional treatments.

### **About MB-101 (IL13R $\alpha$ 2-specific CAR T cells)**

IL13R $\alpha$ 2 is an attractive target for CAR T therapy as it has limited expression in normal tissue but is over-expressed on the surface of the majority of GBM. CAR T cells are designed to express a membrane-tethered IL-13 receptor ligand (IL-13) incorporating a single point mutation that provides high affinity for IL13R $\alpha$ 2 and reduces binding to IL13R $\alpha$ 1 in order to reduce healthy tissue targeting.

Mustang is developing an optimized CAR T product incorporating enhancements in CAR design and T cell engineering to improve antitumor potency and T cell persistence. We include a second-generation hinge optimized CAR containing mutations in the IgG4 linker to reduce off-target Fc interactions, as well as the 41BB (CD137) co-stimulatory signaling domain for improved persistence of CAR T cells, and extracellular domain of CD19 as a selection/safety marker. In order to further improve persistence, central memory T cells are enriched and genetically engineered using a manufacturing process that limits ex vivo expansion in order to reduce T cell exhaustion and maintain a memory T cell phenotype.

### **About Mustang Bio**

Mustang Bio, Inc., a Fortress Biotech Company, is a clinical-stage biopharmaceutical company focused on the development and commercialization of novel cancer immunotherapy products designed to utilize the power of the patient's own immune system to eliminate cancer cells. Mustang aims to acquire rights to these technologies by licensing or otherwise acquiring an ownership interest in the technologies, funding their research and development, and out-licensing or bringing the technologies to market. Mustang is currently developing proprietary Chimeric Antigen Receptor (CAR) engineered T cells (CAR T) technology, which was licensed from Drs. Stephen Forman and Christine Brown's laboratory at the City of Hope National Medical Center (COH). CAR T uses the patient's own T cells to engage and destroy specific tumors. The process involves selecting specific T cell subtypes, genetically engineering them to express chimeric antigen T cell receptors and placing them back in the patient where they recognize and destroy cancer cells. Mustang, through a research agreement with COH, plans to develop CARs across multiple cancers. Its lead programs in acute myeloid leukemia and brain cancer are in Phase 1 clinical trials. Mustang is registered under the Securities Exchange Act of 1934, as amended, and files

periodic reports with the US Securities and Exchange Commission. For more information, visit [www.mustangbio.com](http://www.mustangbio.com).

**Mustang Bio is a majority-owned subsidiary of Fortress Biotech.**

**About Fortress Biotech**

Fortress Biotech, Inc. (“Fortress”) is a biopharmaceutical company dedicated to acquiring, developing and commercializing novel pharmaceutical and biotechnology products. Fortress develops and commercializes products both within Fortress and through certain of its subsidiary companies, also known as Fortress Companies. Additionally, Fortress recently acquired a controlling interest in National Holdings Corporation (NASDAQ: NHLD), a diversified independent brokerage company (together with its subsidiaries, “NHLD”). In addition to its internal development programs, Fortress leverages its biopharmaceutical business expertise and drug development capabilities and provides funding and management services to help the Fortress Companies achieve their goals. Fortress and the Fortress Companies may seek licensings, acquisitions, partnerships, joint ventures and/or public and private financings to accelerate and provide additional funding to support their research and development programs. For more information, visit [www.fortressbiotech.com](http://www.fortressbiotech.com).

**Forward-Looking Statements**

This press release may contain “forward-looking statements” within the meaning of Section 27A of the Securities Act of 1933 and Section 21E of the Securities Exchange Act of 1934. Such statements include, but are not limited to, any statements relating to our growth strategy and product development programs and any other statements that are not historical facts. Forward-looking statements are based on management’s current expectations and are subject to risks and uncertainties that could negatively affect our business, operating results, financial condition and stock price. Factors that could cause actual results to differ materially from those currently anticipated include: risks related to our growth strategy; risks relating to the results of research and development activities; our ability to obtain, perform under and maintain financing and strategic agreements and relationships; uncertainties relating to preclinical and clinical testing; our dependence on third party suppliers; our ability to attract, integrate, and retain key personnel; the early stage of products under development; our need for substantial additional funds; government regulation; patent and intellectual property matters; competition; as well as other risks described in our SEC filings. We expressly disclaim any obligation or undertaking to release publicly any updates or revisions to any forward looking statements contained herein to reflect any change in our expectations or any changes in events, conditions or circumstances on which any such statement is based, except as may be required by law.

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