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## **Mustang Bio and City of Hope Announce Initiation of Phase 1 Clinical Trial of MB-101 (IL13R $\alpha$ 2-specific CAR T cells) to Treat Leptomeningeal Brain Tumors**

**Trial underway at City of Hope to evaluate safety and feasibility of administering therapy in patients with brain tumors such as glioblastoma, ependymoma or medulloblastoma**

WORCESTER, Mass., Dec. 21, 2020 (GLOBE NEWSWIRE) -- Mustang Bio, Inc. ("Mustang") (NASDAQ: MBIO), a clinical-stage biopharmaceutical company focused on translating today's medical breakthroughs in cell and gene therapies into potential cures for hematologic cancers, solid tumors and rare genetic diseases, and City of Hope, a world-renowned independent research and treatment center for cancer, diabetes and other life-threatening diseases, today announced that a Phase 1 single-center, two-arm clinical trial has been initiated to establish the safety and feasibility of administering MB-101 (autologous IL13R $\alpha$ 2-CAR T cells) to patients with leptomeningeal brain tumors (e.g., glioblastoma, ependymoma or medulloblastoma). The trial will enroll up to 30 patients and take place at City of Hope, where the chimeric antigen receptor T ("CAR T") cell therapy was initially developed.

All subjects enrolled in the trial will undergo surgery for the placement of an intraventricular (ICV) Rickham catheter for CAR T cell delivery. The Phase 1 trial will establish the safety and feasibility of administering MB-101 through the ICV Rickham catheter over four weekly cycles in patients with glioblastoma (Arm 1) and ependymoma or medulloblastoma (Arm 2). The primary endpoints that will be evaluated are toxicity and survival at three months. Secondary endpoints include overall survival, CAR T and endogenous T cell levels, cytokine levels and phenotype detection in peripheral blood, tumor cyst fluid and cerebrospinal fluid.

[Lisa Feldman](#), M.D., Ph.D., a neurosurgeon and assistant clinical professor in the Division of Neurosurgery at City of Hope and principal investigator of the clinical trial, commented, "Leptomeningeal brain tumors are a form of metastatic brain cancer, which is currently very difficult to treat. We are encouraged by the potential of administering autologous IL13R $\alpha$ 2-CAR T cells intraventricularly to patients with leptomeningeal brain tumors. This CAR T cell therapy has demonstrated early safety and efficacy results in a previous clinical trial conducted at City of Hope, and we believe these preliminary results warrant further

evaluation of these CAR T cells. We look forward to providing updates on the trial and to continue working closely with Mustang with the goal of bringing a safe and effective treatment option to patients with this life-threatening disease.”

Manuel Litchman, M.D., president and chief executive officer of Mustang, said, “We are pleased to further study MB-101 in leptomeningeal brain tumors as it has already demonstrated therapeutic potential when infused into the ventricular system, including delivering a complete response in a leptomeningeal glioblastoma patient that was published in the *New England Journal of Medicine*. Our ongoing work with City of Hope continues to advance the research of our CAR T portfolio to bring potential therapies to patients suffering from devastating diseases.”

Additional information about the trial can be found on [clinicaltrials.gov](https://clinicaltrials.gov) using the identifier [NCT04661384](https://clinicaltrials.gov/ct2/show/study/NCT04661384).

### **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 overexpressed on the surface of the majority of malignant glioma cells, including glioblastoma multiforme, ependymoma and medulloblastoma. 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 MB-101 as an optimized CAR T product incorporating enhancements in CAR design and T cell engineering to improve antitumor potency and T cell persistence. MB-101 includes a second-generation hinge optimized CAR containing mutations in the IgG4 linker to reduce off-target Fc interactions, the 4-1BB (CD137) co-stimulatory signaling domain for improved persistence of CAR T cells and the extracellular domain of CD19 as a selection/safety marker. To further improve persistence, central memory T cells are enriched and genetically engineered using a manufacturing process that limits *ex vivo* expansion to reduce T cell exhaustion and maintain a memory T cell phenotype.

### **About Mustang Bio**

Mustang Bio, Inc. is a clinical-stage biopharmaceutical company focused on translating today’s medical breakthroughs in cell and gene therapies into potential cures for hematologic cancers, solid tumors and rare genetic diseases. Mustang aims to acquire rights to these technologies by licensing or otherwise acquiring an ownership interest, to fund research and development and to outlicense or bring the technologies to market. Mustang has partnered with top medical institutions to advance the development of CAR T therapies across multiple cancers, as well as a lentiviral gene therapy for X-linked severe combined immunodeficiency (XSCID), also known as bubble boy disease. Mustang is registered under the Securities Exchange Act of 1934, as amended, and files periodic reports with the U.S. Securities and Exchange Commission (“SEC”). Mustang was founded by Fortress Biotech, Inc. (NASDAQ: FBIO). For more information, visit [mustangbio.com](https://mustangbio.com).

### **About City of Hope**

City of Hope is an independent biomedical research and treatment center for cancer, diabetes and other life-threatening diseases. Founded in 1913, City of Hope is a leader in [bone marrow transplantation](#) and immunotherapy such as [CAR T cell therapy](#). City of Hope’s translational research and personalized treatment protocols advance care throughout the world. Human synthetic insulin and [numerous breakthrough cancer](#)

[drugs](#) are based on technology developed at the institution. A National Cancer Institute-designated comprehensive cancer center and a founding member of the National Comprehensive Cancer Network, City of Hope has been ranked among the nation's "Best Hospitals" in cancer by U.S. News & World Report for 14 consecutive years. Its main campus is located near Los Angeles, with [additional locations](#) throughout Southern California. For more information about City of Hope, follow us on [Facebook](#), [Twitter](#), [YouTube](#) or [Instagram](#).

### **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, each as amended. 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 value. Factors that could cause actual results to differ materially from those currently anticipated include: risks relating to our growth strategy; our ability to obtain, perform under, and maintain financing and strategic agreements and relationships; risks relating to the results of research and development activities; risks relating to the timing of starting and completing clinical trials; 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 required by law, and we claim the protection of the safe harbor for forward-looking statements contained in the Private Securities Litigation Reform Act of 1995.

### **Company Contacts:**

Jaclyn Jaffe and William Begien  
Mustang Bio, Inc.  
(781) 652-4500  
[ir@mustangbio.com](mailto:ir@mustangbio.com)

### **Investor Relations Contact:**

Daniel Ferry  
LifeSci Advisors, LLC  
(617) 430-7576  
[daniel@lifesciadvisors.com](mailto:daniel@lifesciadvisors.com)

### **Media Relations Contacts:**

Tony Plohoros  
6 Degrees  
(908) 591-2839  
[tplohoros@6degreespr.com](mailto:tplohoros@6degreespr.com)

Letisia Marquez  
City of Hope

(626) 476-7593

[lemarquez@coh.org](mailto:lemarquez@coh.org)



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