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# Mustang Bio's MB-102 (CD123 CAR) CAR T Therapy Achieves Complete Response in Acute Myeloid Leukemia and Blastic Plasmacytoid Dendritic Cell Neoplasm in Phase 1 Clinical Trial

*Trial investigators at City of Hope announce first-reported complete response from a CAR T therapy in a BPDCN patient, additional complete response achieved in AML*

*Investigators found infusions of MB-102 are safe and well tolerated*

*Data presented by City of Hope in oral session at ASH Annual Meeting*

NEW YORK, Dec. 11, 2017 (GLOBE NEWSWIRE) -- Mustang Bio, Inc. ("Mustang") (NASDAQ:MBIO), a Fortress Biotech (NASDAQ:FBIO) Company focused on the development of novel immunotherapies based on proprietary chimeric antigen receptor engineered T cell ("CAR T") technology, today announced that investigators at City of Hope have reported that Mustang's MB-102 (CD123 CAR) CAR T therapy is safe and well tolerated and achieved the first-ever complete response (CR) from a CAR T therapy in blastic plasmacytoid dendritic cell neoplasm (BPDCN), as well as a CR in acute myeloid leukemia (AML), in an ongoing Phase 1 clinical trial ([NCT02159495](https://clinicaltrials.gov/ct2/show/study/NCT02159495)). The data were presented by City of Hope today in an oral session at the 59th American Society of Hematology (ASH) Annual Meeting.

Manuel Litchman, M.D., President and Chief Executive Officer of Mustang, said, "According to investigators at City of Hope, these data demonstrate MB-102's potential to be a safe, well tolerated and effective CAR T therapy that can achieve complete disease response. In addition, MB-102's promising anti-leukemic activity in both AML and BPDCN supports further evaluation in clinical trials in transplant eligible and ineligible patients. We are thrilled to report that our CAR Ts have now achieved complete responses in three disease areas, MB-102 in AML and BPDCN, and our MB-101 IL13R $\alpha$ 2-specific CAR T in glioblastoma, which was published in December 2016 in the *New England Journal of Medicine*."

Elizabeth Lihua Budde, M.D., Ph.D., assistant professor in the department of Hematology & Hematopoietic Cell Transplantation at City of Hope and principal investigator for the Phase 1 trial, said, "Current treatment options in AML are associated with low rates of complete

response and limited progression to allogeneic hematopoietic stem cell transplantation. Moreover, BPDCN is a rare and incurable blood cancer with no standard of care. CD123 is overexpressed in both AML and BPDCN, making it an attractive target in these diseases, which have clear unmet therapeutic needs. We are encouraged by these interim data that demonstrate MB-102's potential to be a new or improved treatment option in BPDCN and AML, and look forward to continuing to evaluate the clinical benefits of MB-102 in our ongoing Phase 1 clinical trial."

### **Key Efficacy and Safety Findings**

This single center, first-in-human Phase 1 dose-escalation clinical trial is evaluating the safety and activity of escalating doses of MB-102 in patients with relapsed or refractory AML (cohort 1) and BPDCN (cohort 2). Patients receive a single dose of MB-102 with an option for a second infusion if they continue to meet safety and eligibility criteria and still have CD123+ disease. To date, 14 patients have been enrolled and seven have been treated (six with AML, one with BPDCN) in this first in-human trial for AML and BPDCN patients using a CD123 CAR T therapy.

In the AML cohort, two patients were treated at dose level 1 (50M CAR+ T). Trial investigators reported that one achieved a morphologic leukemic-free state at day 28 post-infusion. Four patients received dose level 2 (200M CAR+ T), with a CR observed at day 28 in one patient, and a CR with incomplete blood count recovery demonstrated at day 28 in a second patient. Both patients proceeded to a second allogeneic hematopoietic stem cell transplantation.

In the BPDCN cohort, one patient received a single dose of 100M CAR+ T and achieved a CR at day 28, which lasted at least 60 days, according to investigators. Of note, this patient had previously experienced disease progression following five cycles of treatment with a CD123-targeted recombinant fusion protein.

Investigators found MB-102 infusions of up to 200M CAR T cells were safe, with no graft-versus-host disease, myeloablative effects, neurologic toxicity or dose-limiting toxicities. Adverse events (AEs) included: cytokine release syndrome (six grade 1, one grade 2), neurotoxicity (dizziness: one grade 1, two grade 2; headache: five grade 1, two grade 2; somnolence: one grade 1, two grade 2), three cases of infection (lung infection: two, other: one). The most common  $\geq$  grade 3 AEs included lymphopenia (seven), thrombocytopenia (seven) and febrile neutropenia (six).

### **About Acute Myeloid Leukemia**

Acute myeloid leukemia (AML) is a cancer of the myeloid line of blood cells characterized by rapid growth of abnormal white blood cells that accumulate in the bone marrow. Although AML is rare, there are approximately 20,000 new cases in the U.S. each year and 10,000 deaths. Current treatment of relapsed or refractory AML with chemotherapy or hematopoietic stem cell transplantation is associated with low rates of complete response and considerable complications.

CD123 is overexpressed on AML blasts and leukemic stem cell-enriched cell subpopulations compared to normal hematopoietic stem cells and myeloid progenitors, making CD123 an attractive target for T cell-based adoptive immunotherapy.

### **About Blastic Plasmacytoid Dendritic Cell Neoplasm**

Blastic plasmacytoid dendritic cell neoplasm (BPDCN) is a rare and incurable blood cancer with a median survival of less than 18 months and no standard of care. High levels of CD123 expression is one of the diagnostic hallmarks of BPDCN, making CD123 an attractive target for T cell-based adoptive immunotherapy.

### **About MB-102 (CD123 CAR)**

MB-102 (CD123CAR) is a CAR T cell therapy that engineers patient T cells to recognize and eliminate CD123-expressing tumors. CD123 is widely expressed on human hematologic malignancies including acute myeloid leukemia (AML), B cell acute lymphoblastic leukemia, hairy cell leukemia, blastic plasmacytoid dendritic cell neoplasm (BPDCN), chronic myeloid leukemia and Hodgkin's lymphoma.

MB-102 has demonstrated anti-AML activity in preclinical studies (Mardiros, *Blood* 2013), and is currently being evaluated in a Phase 1 clinical trial in AML and BPDCN at City of Hope ([NCT02159495](https://clinicaltrials.gov/ct2/show/study/NCT02159495)).

### **About Mustang Bio**

Mustang Bio, Inc., a subsidiary of Fortress Biotech, Inc., is a clinical-stage biopharmaceutical company focused on the development and commercialization of novel cancer immunotherapy products designed to leverage 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, funding research and development, and outlicensing or bringing the technologies to market. Mustang has partnered with the City of Hope National Medical Center ("COH") and the Fred Hutchinson Cancer Research Center in the development of proprietary chimeric antigen receptor ("CAR") engineered T cell ("CAR T") therapies across many cancers, and with Harvard Medical School's Beth Israel Deaconess Medical Center and the Harvard Stem Cell Institute for the development of CRISPR/Cas9-enhanced CAR T therapies in hematologic malignancies and solid tumors. Mustang's lead programs are in Phase 1 clinical trials at COH: MB-101 for the treatment of brain cancer and MB-102 as a therapeutic agent in acute myeloid leukemia and blastic plasmacytoid dendritic cell neoplasm. Mustang is registered under the Securities Exchange Act of 1934, as amended, and files periodic reports with the U.S. Securities and Exchange Commission. For more information, visit [www.mustangbio.com](http://www.mustangbio.com).

### **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. 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 licensing arrangements, 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,

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.

**Contacts:**

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

Fortress Biotech Media Relations  
Laura Bagby  
6 Degrees  
(312) 448-8098  
[lbagby@6degreespr.com](mailto:lbagby@6degreespr.com)



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