



## **Mustang Bio Receives Orphan Drug Designation for MB-102 (CD123 CAR T) for the Treatment of Acute Myeloid Leukemia**

**New York, NY – July 24, 2019** – 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, today announced that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation to MB-102 (CD123 CAR T) for the treatment of acute myeloid leukemia (AML). The FDA also previously granted Orphan Drug Designation to MB-102 (CD123 CAR T) for the treatment of blastic plasmacytoid dendritic cell neoplasm (BPDCN).

Manuel Litchman, M.D., President and Chief Executive Officer of Mustang, said, “We are pleased that MB-102 has received Orphan Drug Designation in two indications, AML and BPDCN. AML most commonly occurs in senior adults, many of whom have to forgo chemotherapy due to other health conditions. MB-102 has the potential to be an important new treatment for these and other patients, and to potentially address multiple areas of high unmet medical need. We expect to initiate a multicenter Phase 1/2 clinical trial in patients with AML, BPDCN and high-risk myelodysplastic syndrome in the coming months.”

MB-102 is currently being studied in a City of Hope, first-in-human Phase 1 dose-escalation clinical trial evaluating the safety and anti-tumor 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. MB-102 has demonstrated complete responses at low doses in AML and BPDCN without dose-limiting toxicities. City of Hope developed CD123 CAR T.

The FDA grants Orphan Drug Designation to drugs and biologics that are intended for the safe and effective treatment, diagnosis or prevention of rare diseases or disorders that affect fewer than 200,000 people in the U.S. Orphan Drug Designation provides certain incentives, such as tax credits toward the cost of clinical trials and prescription drug user fee waivers. If a product holding Orphan Drug Designation receives the first FDA approval for the disease in which it has such designation, the product is entitled to seven years of market exclusivity, which is independent from intellectual property protection.

### **About Acute Myeloid Leukemia**

AML is a cancer of the myeloid line of blood cells, characterized primarily by the rapid growth of abnormal white blood cells that build up in the bone marrow and interfere with the production of normal blood cells. CD123 is an attractive target for T cell-based adoptive immunotherapy due to high levels of CD123 expression in AML.

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

MB-102 (CD123 CAR T) is a CAR T cell therapy that is produced by engineering patient T cells to recognize and eliminate CD123-expressing tumors. CD123 is widely expressed on bone marrow cells of patients with myelodysplastic syndromes, as well as in hematologic malignancies, including AML, B-cell acute lymphoblastic leukemia, hairy cell leukemia, BPDCN, chronic myeloid leukemia and Hodgkin’s lymphoma.

In the first-in-human clinical trial at City of Hope ([NCT02159495](#)), MB-102 has demonstrated complete responses at low doses in AML and BPDCN without dose-limiting toxicities, as reported at the American Society of Hematology (ASH) Annual Meeting in December 2017 and the American Association for Cancer Research (AACR) Special Conference on Tumor Immunology and Immunotherapy in November 2018. Dose escalation continues at City of Hope in both indications.

### **About Mustang Bio**

Mustang Bio, Inc. (“Mustang”) 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 and CRISPR/Cas9-enhanced CAR T therapies across multiple cancers, as well as a lentiviral gene therapy for XSCID. Mustang is registered under the Securities Exchange Act of 1934, as amended, and files periodic reports with the U.S. Securities and Exchange Commission. Mustang was founded by Fortress Biotech, Inc. (NASDAQ: FBIO). For more information, visit [www.mustangbio.com](http://www.mustangbio.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.

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