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# Mustang Bio Receives Orphan Drug Designation for MB-102 (CD123 CAR T) for the Treatment of Blastic Plasmacytoid Dendritic Cell Neoplasm

NEW YORK, Dec. 20, 2018 (GLOBE NEWSWIRE) -- Mustang Bio, Inc. (“Mustang”) (NASDAQ: MBIO), a company focused on the development of novel immunotherapies based on proprietary chimeric antigen receptor engineered T cell (CAR T) technology and gene therapies for rare 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 blastic plasmacytoid dendritic cell neoplasm (BPDCN), a rare and incurable blood cancer with a median survival of less than 18 months and no standard of care.

Martina Sersch, M.D., Ph.D., Chief Medical Officer of Mustang, said, “We are pleased to receive Orphan Drug Designation for MB-102, which has shown the potential to address an area of high unmet medical need. This significant milestone for Mustang will provide additional market exclusivity and financial benefits to advance MB-102, which we believe is an important new treatment for patients with BPDCN. Based on the Phase 1 data presented 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, we expect to initiate a multicenter Phase 1/2 clinical trial in patients with acute myeloid leukemia (AML), BPDCN and high-risk myelodysplastic syndrome in 2019.”

MB-102 is currently being studied in a single center, 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.

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 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 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"), a Fortress Biotech (NASDAQ: FBIO) company, is a clinical-stage biopharmaceutical company focused on the development and commercialization of a broad range of proprietary chimeric antigen receptor engineered T cell (CAR T) immunotherapies and gene therapies in areas of unmet need. 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 X-SCID. 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.

**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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