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## **Mustang Bio Provides Updates on its Lentiviral Gene Therapies for the Treatment of X-linked Severe Combined Immunodeficiency (“XSCID”)**

*FDA removes clinical hold for pivotal Phase 2 MB-107 clinical trial*

*Company plans to enroll first patient in MB-107 pivotal trial in the second quarter of 2021*

*Clinical outcomes in investigator-IND XSCID trials continue to show compelling efficacy*

WORCESTER, Mass., Feb. 02, 2021 (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, today provided updates on MB-107 and MB-207, its lentiviral gene therapies for the treatment of X-linked severe combined immunodeficiency (“XSCID”), also known as bubble boy disease. On January 28, 2021, the U.S. Food and Drug Administration (“FDA”) removed the clinical hold on the MB-107 pivotal Phase 2 clinical trial Investigational New Drug (“IND”) application after reviewing a comprehensive CMC package that was submitted by Mustang in late December 2020. Mustang will proceed with its plans to initiate the pivotal Phase 2 trial in newly diagnosed XSCID patients.

The same lentiviral vector used in MB-107 is currently being assessed in a Phase 1/2 clinical trial for XSCID in newly diagnosed infants under the age of two at St. Jude Children’s Research Hospital (“St. Jude”), UCSF Benioff Children’s Hospital in San Francisco and Seattle Children’s Hospital. Additionally, it is being assessed in a Phase 1/2 clinical trial at the National Institute of Allergy and Infectious Diseases (“NIAID”), part of the National Institutes of Health, for XSCID patients who have been previously treated with hematopoietic stem cell transplantation (“HSCT”) and for whom re-treatment is indicated.

Manuel Litchman, M.D., President and Chief Executive Officer of Mustang, said, “We are pleased to have resolved the clinical hold on the MB-107 IND with the FDA, enabling us to move forward with initiating the pivotal Phase 2 clinical trial. The clinical outcomes observed in XSCID patients in the ongoing Phase 1/2 clinical trials continue to be encouraging. It is especially gratifying to see the consistent safety and efficacy of our lentiviral vector over the course of more than eight years since the first patient was treated at NIAID in 2012. In 2021,

we look forward to the anticipated initiation of our pivotal MB-107 and MB-207 clinical trials as we work to bring potential new treatment options for this devastating rare disease to patients and their families.”

### **MB-107 Update**

Data from the Phase 1/2 clinical trial led by St. Jude that were presented at the 61st American Society of Hematology (“ASH”) Annual Meeting in December 2019 included 11 newly diagnosed XSCID patients who had been treated with a median follow-up at data cut-off of 23.6 months (range 1.5 to 33.9 months). No serious adverse events related to treatment were reported other than hematologic ones related to low-dose busulfan conditioning. Nine patients, with a follow up of greater than 3 months, achieved normal-for-age T-cell and natural killer (NK)-cell numbers within 3-4 months post treatment with MB-107. Five patients were off intravenous immunoglobulin (IVIG) therapy, of whom 3 responded to vaccines.

To date, all 11 patients have continued to do well, and 5 additional patients were enrolled at the time of the most recent analysis in early September 2020. At that time, follow-up for these 16 patients ranged from 3 months to 47 months. Similar to previous reports, the therapy continued to be well tolerated in all patients, and stable vector marking was noted in all lineages, with successful engraftment of genetically-modified T-, B-, & NK-cells. All patients cleared pre-existing infections, no new severe infections were noted, and all patients were outpatients. Finally, there was no evidence of malignant transformation at a median follow up of 2 years. Enrollment will continue at all three clinical sites until Mustang initiates its multicenter pivotal Phase 2 trial of MB-107.

In September 2020, [\*The Journal of Allergy and Clinical Immunology: In Practice\*](#) published a case study of one patient with XSCID and disseminated Bacille Calmette-Guérin (BCG) infection, who was enrolled in the clinical trial at St. Jude. After 2.5 years of treatment, the patient has remained clinically well with a stable, functional immune system and protective vaccine titers, despite the complication of the disseminated BCG infection.

### **MB-207 Update**

The ongoing Phase 1/2 clinical trial being conducted by the NIH is treating older XSCID patients, all of whom had previously received haplo-identical HSCT as infants and were subsequently noted to be experiencing declining immune function with symptomatic infections. At the time of the most recent NIH data presentation at ASH in 2019, 8 patients had been treated without transduction enhancers (referred to as Cohort A) and had been followed for 3 to 7 years. Seven of these 8 patients experienced gradual clinical benefit in terms of clearance of chronic norovirus and associated improved abdominal complaints, malabsorption, growth and IgG production. One of these 7 patients died 27 months after gene therapy of a pulmonary bleed related to chronic bronchiectasis that predated the therapy and was deemed to be unrelated to therapy.

In an attempt to address the relatively slow resolution of chronic norovirus observed in most of these 7 patients and the delayed immune cell recovery and persistent clinical disease in the eighth patient, transduction enhancers were introduced in the cell processing for the subsequent 6 patients (referred to as Cohort B), which included retreatment of the eighth patient in Cohort A who had delayed immune recovery and persistent clinical disease. This enhanced transduction procedure achieved much greater transduction efficiencies than were observed in Cohort A, with greater than 10-fold less vector, and resulted in faster immune

reconstitution and more significant clinical benefit by 3 months. As a result, Mustang has licensed Sirion Biotech's Lentiboost™ and will include transduction enhancement in its pivotal Phase 2 clinical trial for MB-207 in this patient population.

To date, of the 6 Cohort A patients who were alive at the time of the 2019 NIH data readout and who did not undergo repeat therapy, 3 patients have been able to discontinue chronic intravenous immunoglobulin (IVIG) and have experienced sustained restoration of humoral responses to immunization. The remaining 3 patients have had reduced IVIG requirements. All chronic norovirus infections have resolved, and the quality of life of all patients has improved significantly.

The original 6 patients in Cohort B also continue to do well, with longest follow-up now 22 months. Two additional patients have been successfully treated with transduction enhancers, for a total of 8 patients in Cohort B. As was the case in Cohort A, no serious adverse events related to treatment were reported other than hematologic related to low-dose busulfan conditioning, and there was no evidence of malignant transformation. Further enrollment at NIH is now limited pending Mustang's initiation of its pivotal multicenter Phase 2 clinical trial, and the company expects to submit an Investigational New Drug ("IND") application for this trial in the second quarter of 2021.

### **About X-linked Severe Combined Immunodeficiency ("XSCID")**

X-linked severe combined immunodeficiency is a rare genetic disorder that occurs in approximately 1 per 225,000 births. It is characterized by the absence or lack of function of key immune cells, resulting in a severely compromised immune system and death by 1 year of age if untreated. Patients with XSCID have no T-cells or natural killer (NK)-cells. Although their B-cells are normal in number, they are not functional. As a result, XSCID patients are usually affected by severe bacterial, viral or fungal infections early in life and often present with interstitial lung disease, chronic diarrhea and failure to thrive. Among patients who receive HSCT, many are unable to establish adequate T-cell immunity or lose T-cell immunity over time. Further, approximately two-thirds of patients who receive HSCT lack sufficient B-cell immunity and need lifelong immunoglobulin replacement therapy.

The specific genetic disorder that causes XSCID is a mutation in the gene coding for the common gamma chain ( $\gamma$ c), a protein that is shared by the receptors for at least six interleukins. These interleukins and their receptors are critical for the development and differentiation of immune cells. The gene coding for  $\gamma$ c is known as IL-2 receptor gamma, or *IL2RG*. Because *IL2RG* is located on the X-chromosome, XSCID is inherited in an X-linked recessive pattern, resulting in almost all patients being male.

### **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 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 ("SEC"). 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, and we claim the protection of the safe harbor for forward-looking statements contained in the Private Securities Litigation Reform Act of 1995.

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