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Mustang Bio Announces Rare Pediatric Disease Designation for MB-107 for the Treatment of X-linked Severe Combined Immunodeficiency

WORCESTER, Mass., Aug. 17, 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, today announced that the U.S. Food and Drug Administration ("FDA") has granted Rare Pediatric Disease Designation to MB-107, Mustang's lentiviral gene therapy for the treatment of X-linked severe combined immunodeficiency ("XSCID"), also known as bubble boy disease, in newly-diagnosed infants. The FDA previously granted Regenerative Medicine Advanced Therapy designation to MB-107 for the treatment of XSCID in newly-diagnosed infants in August 2019. Additionally, the European Medicines Agency granted Advanced Therapy Medicinal Product classification to MB-107 in April 2020.

The FDA grants Rare Pediatric Disease Designation for serious and life-threatening diseases that primarily affect children ages 18 years or younger and affect fewer than 200,000 people in the United States. If Mustang's Biologics License Application is approved, the company may be eligible to receive a priority review voucher, which can be redeemed to obtain priority review for any subsequent marketing application and may be sold or transferred. This program is intended to encourage development of new drugs and biologics for the prevention and treatment of rare pediatric diseases.

Manuel Litchman, M.D., President and Chief Executive Officer of Mustang, said, "We are very pleased that the FDA has granted Rare Pediatric Disease Designation to MB-107 for XSCID, a life-threatening and rare genetic disorder with limited treatment options, in newly-diagnosed infants. We anticipate that our pivotal clinical program will begin shortly, and we look forward to continuing to work efficiently with the FDA, in order to bring MB-107 to children suffering from this devastating disease as quickly and safely as possible."

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. In May 2020, Mustang submitted an investigational new drug application ("IND") to the FDA to initiate a multi-center Phase 2 clinical trial of MB-107 in newly diagnosed infants with

XSCID who are between two months to two years of age. The trial is expected to enroll 10 patients who, together with 15 patients enrolled in the current multi-center trial led by St. Jude, will be compared with 25 matched historical control patients who have undergone hematopoietic stem cell transplantation ("HSCT"). The primary efficacy endpoint will be event-free survival. The initiation of this trial is expected in early Q4 2020. Mustang is targeting topline data from this trial in the second half of 2022. Another Phase 1/2 clinical trial for XSCID in patients over the age of two years, who have received prior HSCT, is underway at the National Institutes of Health, and Mustang expects to file an IND to the FDA to initiate a multi-center Phase 2 clinical trial in this population in Q4 2020. The product in this second trial will be designated MB-207.

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 one year of age if untreated. Patients with XSCID have no T-cells or natural killer 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.

The specific genetic disorder that causes XSCID is a mutation in the gene coding for the common gamma chain ("γc"), 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 γc 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.

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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