

September 2, 2020



# **Mustang Bio Announces Orphan Drug Designation for MB-107 for the Treatment of X-linked Severe Combined Immunodeficiency in Newly Diagnosed Infants**

WORCESTER, Mass., Sept. 02, 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 Orphan Drug 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 under the age of two. The FDA previously granted Rare Pediatric Disease Designation in August 2020 and Regenerative Medicine Advanced Therapy designation in August 2019 to MB-107 for the treatment of XSCID in newly diagnosed patients. Additionally, the European Medicines Agency granted Advanced Therapy Medicinal Product classification to MB-107 in April 2020.

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.

Manuel Litchman, M.D., President and Chief Executive Officer of Mustang, said, "Mustang has had a productive quarter on the regulatory front. We are very pleased to achieve another significant milestone and receive Orphan Drug Designation for MB-107 for the treatment of XSCID in newly diagnosed patients. This designation for MB-107, in addition to its Rare Pediatric Disease Designation, Regenerative Medicine Advanced Therapy designation and Advanced Therapy Medicinal Product classification, continues to enhance our regulatory pathway for a much-needed treatment option to address this devastating rare disease that affects children. We look forward to initiating our pivotal clinical programs for MB-107 in newly diagnosed infants with XSCID and MB-207 in previously transplanted

patients with XSCID very soon.”

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 early in the fourth quarter of 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 the fourth quarter of 2020. This product candidate for XSCID in patients over the age of two years, who have received prior HSCT, is designated MB-207. The FDA granted a Rare Pediatric Disease Designation to MB-207 in August 2020.

### **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 (“ $\gamma$ 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.

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Source: Mustang Bio, Inc.