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# Mustang Bio Announces Positive Opinion from the European Medicines Agency on Orphan Drug Designation for Its Lentiviral Gene Therapy for the Treatment of X-linked Severe Combined Immunodeficiency (“XSCID”)

WORCESTER, Mass., Nov. 24, 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 European Commission (“EC”) issued a positive opinion on its application for Orphan Drug Designation for Mustang’s lentiviral gene therapy for the treatment of X-linked severe combined immunodeficiency (“XSCID”), also known as bubble boy disease. The Designation applies both to MB-107 for the treatment of newly diagnosed infants between two months and two years of age and to MB-207 for the treatment of patients who have been previously treated with hematopoietic stem cell transplantation (“HSCT”) and for whom re-treatment is indicated. The European Medicines Agency (“EMA”) previously granted Advanced Therapy Medicinal Product classification to MB-107 in April 2020. The U.S. Food and Drug Administration (“FDA”) also previously granted Rare Pediatric Disease and Orphan Drug Designations to MB-107 and MB-207, as well as Regenerative Medicine Advanced Therapy Designation to MB-107.

Orphan Drug Designation in the European Union (“EU”) is granted by the European Commission based on a positive opinion issued by the European Medicines Agency Committee for Orphan Medicinal Products (EMA COMP). To qualify, an investigational medicine must be intended to treat a seriously debilitating or life-threatening condition that affects fewer than five in 10,000 people in the EU, and there must be sufficient non-clinical or clinical data to suggest the investigational medicine may produce clinically relevant outcomes. EMA Orphan Drug Designation provides companies with certain benefits and incentives, including protocol assistance, differentiated evaluation procedures for Health Technology Assessments in certain countries, access to a centralized marketing authorization procedure valid in all EU member states, reduced regulatory fees and 10 years of market exclusivity.

Manuel Litchman, M.D., President and Chief Executive Officer of Mustang, said, “We are very pleased to receive a positive opinion from the EC on Orphan Drug Designation for our lentiviral gene therapy for XSCID. It is an important milestone for Mustang as we approach the initiation of our pivotal MB-107 and MB-207 clinical trials, which we anticipate will support regulatory filings in both the U.S. and EU. We look forward to working closely with the EMA as we continue our progress to make MB-107 and MB-207 available for patients suffering with XSCID.”

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. Mustang submitted an investigational new drug application (“IND”) to the FDA to initiate a pivotal multi-center Phase 2 clinical trial of MB-107 in this same patient population. 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 HSCT. The primary efficacy endpoint will be event-free survival. The initiation of this trial is expected soon. Mustang is targeting topline data from this trial in the second half of 2022.

Earlier this month, Mustang signed an agreement with Minaris Regenerative Medicine GmbH (“Minaris”), a leading contract development and manufacturing service provider for the cell and gene therapy industry, to enable technology transfer and GMP clinical manufacturing of Mustang’s MB-107 lentiviral gene therapy program for the treatment of XSCID in newly diagnosed infants in Europe. Under the terms of the agreement, Minaris will perform technology transfer of the manufacturing and analytical processes, as well as their adoption to the European regulatory environment, for the GMP-compliant manufacturing of the drug product at its site in Ottobrunn, Germany, with the goal of supplying clinical trials in Europe.

MB-207 is currently being assessed in a Phase 1/2 clinical trial at the National Institute of Allergy and Infectious Diseases for XSCID patients who have been previously treated with HSCT and for whom re-treatment is indicated. Mustang expects to file an IND with the FDA to initiate a pivotal multi-center pivotal Phase 2 clinical trial of MB-207 in this same patient population in the first quarter of 2021 and is targeting topline data from this trial in the second half of 2022.

**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, 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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