January 24, 2022

Mustang Bio Provides an Update on its IND Application for MB-207, a Lentiviral Gene Therapy for Treatment of X-linked Severe Combined Immunodeficiency ("XSCID") in Previously Transplanted Patients

WORCESTER, Mass., Jan. 24, 2022 (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 issued a hold, pending Chemistry, Manufacturing and Controls ("CMC") clearance, on the Company's Investigational New Drug ("IND") application. Submitted in December 2021, the IND is to initiate a pivotal Phase 2 multicenter study to assess the safety, tolerability and efficacy of MB-207, Mustang's lentiviral gene therapy for the treatment of patients with X-linked severe combined immunodeficiency ("XSCID"), also known as bubble boy disease, who have been previously treated with a hematopoietic stem cell transplantation ("HSCT") and for whom re-treatment is indicated. The FDA has previously granted MB-207 Orphan Drug and Rare Pediatric Disease Designations. As such, if an MB-207 Biologics License Application ("BLA") is approved by the FDA, then MB-207 would be eligible for a rare pediatric disease voucher.

An additional Phase 1/2 clinical trial for XSCID in newly diagnosed infants under the age of two is ongoing at St. Jude, UCSF Benioff Children's Hospital in San Francisco and Seattle Children's Hospital. The product candidate in this trial is designated as MB-107. Mustang expects to initiate a multi-center pivotal Phase 2 clinical trial of MB-107 under a Mustang-sponsored IND in newly diagnosed infants with XSCID who are between two months to two years of age in the third quarter of 2022. 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. Similar to MB-207, if an MB-107 BLA is approved by the FDA, then MB-107 would be eligible for a rare pediatric disease voucher.

The FDA previously granted Rare Pediatric Disease, Orphan Drug and Regenerative Medicine Advanced Therapy Designations to MB-107 for the treatment of XSCID in newly

diagnosed infants. Additionally, the European Medicines Agency granted Advanced Therapy Medicinal Product Classification, Orphan Drug and PRIME designation to MB-107.

Manuel Litchman, M.D., President and Chief Executive Officer of Mustang, said, “We appreciate the FDA’s support and guidance as we pursue the advancement of MB-107 and MB-207 as potential treatment options for XSCID, a life-threatening, rare genetic disorder with limited treatment options available. We hope to efficiently expedite the development of both critically needed treatments for newly diagnosed infants with XSCID who are between two months to two years of age and for children with XSCID who have previously received HSCT and require re-treatment. We look forward to initiating our multi-center pivotal Phase 2 clinical trial of MB-107 under a Mustang-sponsored IND in newly diagnosed infants with XSCID who are between two months to two years of age in the third quarter of 2022. In light of our positive experience managing the prior MB-107 CMC hold, and our ability to secure FDA clearance to proceed with that program, we believe that our CMC team is well positioned to address the Agency’s concerns around MB-207 once additional clarification of the hold becomes available. Furthermore, we remain fully committed to the success of the pivotal Phase 2 MB-207 clinical trial for children with XSCID who have previously received HSCT and require re-treatment.”

The lentiviral gene-therapy method employed in MB-207 was co-developed by scientists at the National Institute of Allergy and Infectious Diseases (“NIAID”), part of the National Institutes of Health (“NIH”), and St. Jude Children’s Research Hospital (“St. Jude”). MB-207 has been studied at NIAID since 2012 and continues to be assessed in an NIAID-supported Phase 1/2 clinical trial for XSCID in patients over the age of two who have received prior HSCT as infants and have subsequently been noted to be experiencing declining immune function with symptomatic infections.

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 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 (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 lentiviral gene therapies for severe combined immunodeficiency. 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.

Company Contacts:
Jaclyn Jaffe and Bill Begien
Mustang Bio, Inc.
(781) 652-4500
ir@mustangbio.com

Investor Relations Contact:
Daniel Ferry
LifeSci Advisors, LLC
(617) 430-7576
daniel@lifesciadvisors.com

Media Relations Contact:
Tony Plohoros
6 Degrees
(908) 591-2839
tplohoros@6degreespr.com

Source: Mustang Bio, Inc.