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Mustang Bio Announces Data on Treatment with Lentiviral Viral Vector Gene Therapy for X-Linked Severe Combined Immunodeficiency Selected for Oral Presentation at American Society of Gene & Cell Therapy 25th Annual Meeting

Data represent largest cohort of infants with XSCID, also known as bubble boy disease, who received lentiviral gene therapy with the longest follow-up to date

Seventeen of 18 patients with follow-up greater than 6 months achieved robust immune reconstitution

All 23 treated patients are alive and 20 patients with follow-up greater than 4 months recovered from pre-existing infections, are off protective isolation and prophylactic antimicrobials, and have normal growth velocity

WORCESTER, Mass., May 03, 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 interim Phase 1/2 data on treatment with the same lentiviral vector used in MB-107, Mustang’s lentiviral gene therapy for X-linked severe combined immunodeficiency (“XSCID”), also known as bubble boy disease, in newly diagnosed infants under the age of two, were selected for an oral presentation during the Clinical Trials Spotlight Symposium at the American Society of Gene & Cell Therapy (“ASGCT”) 25th Annual Meeting taking place May 16-19, 2022, both virtually and in Washington, D.C.

The presentation will include updated data from a multicenter 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. The lentiviral gene therapy is also 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.

The data include 23 infants with XSCID treated with the lentiviral vector at a median age of 3 months (range: 2.4-13.8) with a median follow-up of 2.4 years (range: 1.4 months to 5.4 years), making it the largest known cohort of infants treated with lentiviral (LV) gene therapy with the longest follow-up. Transduced autologous bone marrow CD34+ cells were generated for all patients with a median vector copy number (VCN) of 0.81/cell (range: 0.16-1.81), and a median CD34+ cell dose of $9.61x10^6$/kg (range 4.4-18.95). Prior to the infusion of cells, patients received busulfan targeted to a cumulative area-under-the-curve (cAUC) of 22 mg*hr/L. All had hematopoietic recovery.

Seventeen of 18 patients with a follow up of >6 months achieved robust immune reconstitution [median CD3+ 2,545/uL (range: 922-4,321), CD4+ 1,568/uL (range: 436-3,556), CD4+/CCR7+/CD45RO- 1,416/uL (range: 298-3,307)]. In these 17 patients, T cells matured appropriately as assessed by normal T cell receptor excision circles (TRECs) and TCRvβ repertoire diversity and were functional as judged by phytohemagglutinin activation. As presented previously in St. Jude's 2019 *New England Journal of Medicine* paper (*N Engl J Med* 2019;380:1525-34.) and the accompanying Supplemental Appendix, the eighteenth patient achieved robust immune reconstitution as well following a gene therapy boost 12 months after the first infusion. Immunoglobulin replacement was discontinued in 15 patients, 12 have been immunized and two more have begun immunizations. Substantial multilineage engraftment occurred in all patients and was sustained over time as judged by VCN analysis in T, B, NK, and myeloid cells separated from peripheral blood. This analysis included 55 samples of 14 patients with ≥1.5 years of follow-up (VCN sample range: 1.5 to 5 years).

All treated patients are alive and 20 patients with a follow-up >4 months recovered from pre-existing infections, are off protective isolation and prophylactic antimicrobials, and have normal growth velocity. Identified integration site hotspots were consistent with previous reports for LV vectors, and no evidence of clonal expansion was observed.

"We're looking forward to the upcoming presentation by St. Jude's Dr. Ewelina Mamcarz at the ASGCT 25th Annual Meeting, as it will highlight updated data representing the largest cohort of infants with XSCID, also known as bubble boy disease, who have been treated with lentiviral gene therapy and the longest follow-up to date," said Manuel Litchman, M.D., President and Chief Executive Officer of Mustang. "Mustang plans to initiate a multicenter pivotal Phase 2 trial for MB-107 under the Company's Investigational New Drug ("IND") application in the second half of this year."

Details of the presentation are as follows:

Title: Lentiviral Gene Therapy with Low Dose Busulfan for Infants with X-Linked Severe Combined Immune Deficiency (XSCID) results in the Development of a Normal and Sustained Immune System: Interim Results of an ongoing Phase I/II Clinical Study
Session: Clinical Trials Spotlight Symposium
Date and Time: Thursday, May 19, 8:45 – 9 a.m. ET
Presenter: Ewelina Mamcarz, M.D., Department of Bone Marrow Transplantation and Cellular Therapy, St. Jude Children's Research Hospital, Memphis, TN

For more information about the ASGCT 25th Annual Meeting, please visit: https://annualmeeting.asgct.org

About X-linked Severe Combined Immunodeficiency ("XSCID")
X-linked severe combined immunodeficiency 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 allogeneic hematopoietic stem cell transplant ("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. XSCID is a rare genetic disorder that occurs in approximately 1 per 225,000 births. There are approximately 2,800 patients with XSCID worldwide who have been previously treated with HSCT, and who therefore might be eligible for gene therapy now or in the future.

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

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