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Mustang Bio Announces Updated Interim Data on X-Linked Severe Combined Immunodeficiency Treatment with Lentiviral Vector Gene Therapy Support Upcoming Multicenter Pivotal Phase 2 Trial for MB-107

All 23 treated patients are alive at 2.6-year median follow-up without evidence of malignant transformation

Data representing largest cohort of infants with XSCID treated with gene therapy presented at the 25th Annual Meeting of the American Society of Gene & Cell Therapy

WORCESTER, Mass., May 19, 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, support 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.

Representing the largest cohort of infants with XSCID treated with gene therapy, the data include 23 infants with XSCID treated with the lentiviral vector at a median age of 3 months (range: 2-14 months) with a median follow-up of 2.6 years (range: 4 months to 5.6 years). 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 9.61×10^6/kg (range 4.40-22.45). Prior to the infusion of cells, patients received busulfan targeted to a cumulative area-under-the-curve (cAUC) of 22 mg*hr/L. The treatment was well tolerated, and all patients experienced complete hematopoietic recovery. Severe adverse events occurred in three patients (two patients with pancytopenia and hemolytic anemia and one patient with delayed neutrophil engraftment), and all resolved. Seventeen patients had active infections prior to therapy, and in all cases these infections cleared. In addition, 15 patients have so far been able to discontinue intravenous immunoglobulin, and

to date 14 patients have been successfully immunized. All patients are currently alive with stable vector marking in all cell lineages and without evidence of malignant transformation.

“We are encouraged by the interim results of this ongoing trial, and our post-treatment follow-up shows that patients continue to do very well,” said Ewelina Mamcarz, M.D., Department of Bone Marrow Transplantation and Cellular Therapy, St. Jude Children’s Research Hospital (“St. Jude”). “The therapy appears to be safe and effective, and the patients in the trial to date have developed functional immune systems with no evidence of abnormal cell division. We plan to continue monitoring these patients for ongoing safety and sustained results, given the limited number of studies where patients have received gene therapy at such a young age.”

The data, presented orally during the Clinical Trials Spotlight Symposium at the 25th Annual Meeting of the American Society of Gene & Cell Therapy (“ASGCT”), are from a multicenter Phase 1/2 clinical trial underway at 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, 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 presented by Dr. Ewelina Mamcarz of St. Jude at ASGCT highlight the ongoing success and potential safety of lentiviral gene therapy treatment for infants with XSCID in the trial conducted to date. In this trial, the treatment has created a stable, functioning immune system for these patients with no sign of leukemogenesis. These data further validate our plan to initiate a multicenter pivotal Phase 2 trial for MB-107 under Mustang’s IND in the second half of this year,” said Manuel Litchman, M.D., President and Chief Executive Officer of Mustang. “We remain hopeful that MB-107 may fill the significant unmet need for a safe and durable treatment for children with bubble boy disease.”

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 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 contains "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, which are often indicated by terms such as "anticipate," "believe," "could," "estimate," "expect," "goal," "intend," "look forward to," "may," "plan," "potential," "predict," "project," "should," "will," "would" and similar expressions, include, but are not limited to, any statements relating to our growth strategy and product development programs, including the timing of and our ability to make regulatory filings such as INDs and other applications and to obtain regulatory approvals for our product candidate, statements concerning the potential of therapies and product candidates, 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 Part I, Item 1A, "Risk Factors," in our Annual Report on Form 10-K filed on March 23, 2022, subsequent Reports on Form 10-Q, and our other filings we make with the 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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