December 9, 2019

Mustang Bio Announces Updated Clinical Data on MB-107 Lentiviral Gene Therapy for Patients with X-Linked Severe Combined Immunodeficiency

MB-107 preceded by low-dose busulfan conditioning continues to be well tolerated and results in development of functional immune system in newly diagnosed infants with XSCID

Enhanced transduction procedure is demonstrating improvements in older patients with XSCID who received prior hematopoietic stem cell transplantation

Data presented by St. Jude Children's Research Hospital and National Institutes of Health at 61st American Society of Hematology Annual Meeting

NEW YORK, Dec. 09, 2019 (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, announced today that updated Phase 1/2 clinical data for MB-107 lentiviral gene therapy for X-linked severe combined immunodeficiency (XSCID) were presented on Saturday by St. Jude Children's Research Hospital ("St. Jude") and today by the National Institutes of Health at the 61st American Society of Hematology (ASH) Annual Meeting.

MB-107 is currently being assessed in two Phase 1/2 clinical trials for XSCID: the first in newly diagnosed infants under the age of two at St. Jude, and the second in patients over the age of two who have received prior hematopoietic stem cell transplantation at the National Institutes of Health. Under a licensing partnership with St. Jude, Mustang intends to develop the lentiviral gene therapy for commercial use as MB-107. The U.S. Food and Drug Administration (FDA) granted Regenerative Medicine Advanced Therapy (RMAT) designation to MB-107 for the treatment of XSCID in August 2019.

Manuel Litchman, M.D., President and Chief Executive Officer of Mustang, said, "The updated clinical data presented at the 2019 ASH Annual Meeting underscore the curative potential of MB-107 for newly diagnosed infants with XSCID, as well as its meaningful impact on older XSCID patients who received prior hematopoietic stem cell transplantation. St. Jude recently received the 2019 Smithsonian Magazine American Ingenuity Award for development of the lentiviral gene therapy, highlighting its potential to have an impact on this

devastating disease. We are excited to be working with St. Jude and NIH to advance MB-107 and look forward to transferring the IND from St. Jude to Mustang in the first quarter of 2020."

Lentiviral Gene Therapy with Low Dose Busulfan for Infants with X-SCID Results in the Development of a Functional Normal Immune System: Interim Results of an Ongoing Phase I/II Clinical Study (Abstract Number: 2058)
Poster presentation: Ewelina Mamcarz, M.D., Department of Bone Marrow Transplantation and Cellular Therapy, St. Jude Children's Research Hospital, Memphis, TN, USA

Interim data from the multicenter Phase 1/2 clinical trial for infants under the age of two treated with the lentiviral gene therapy preceded by low exposure-targeted busulfan conditioning were published in the *New England Journal of Medicine*. Updated data presented at the 2019 ASH Annual Meeting include three more patients (n=11), 8 months additional median follow up (23.6 months; range: 1.5 to 33.9 months), more extensive analysis of T and B cell functional recovery, and detailed vector integration site studies.

Data Highlights:

- Lentiviral gene therapy using low dose busulfan conditioning has been well tolerated, with no serious adverse events other than hematologic related to busulfan
- All 11 patients had robust hematopoietic recovery within 3-4 weeks post cell infusion without blood product support
- Nine patients, with a follow up of greater than 3 months, achieved normal-for-age T-cell and natural killer (NK)-cell numbers within 3-4 months post gene therapy
- Five patients are off intravenous immunoglobulin (IVIG) therapy thus far, of whom 3 responded to vaccines
- Median vector copy number (VCN) at 12 months post-gene therapy in seven patients, who have been followed for more than 12 months, was 2.25 VCN/cell (range: 1.24-3.03) in T cells, 0.34 VCN/cell (range: 0.23-1.25) in B cells, 1.55 VCN/cell (range 1.27-3.39) in NK cells, and 0.08 VCN/cell (range: 0.03-0.76) in myeloid cells in peripheral blood, and 0.10 (range: 0.05-0.66) in CD34+ bone marrow cells, respectively

"The results from treatment with low-dose busulfan conditioning and the novel lentiviral gene therapy in newly diagnosed infants with XSCID continue to be very promising," said Dr. Mamcarz. "We are pleased that the therapy has been well tolerated and all patients with a follow up of more than 3 months recovered from pre-existing infections, are off protective isolation and prophylactic antimicrobials, and have normal growth in respect to height and weight. This reinforces our belief that the lentiviral gene therapy has the potential to be an attractive alternative to current XSCID therapies."

Enhanced Transduction Lentivector Gene Therapy for Treatment of Older Patients with X-Linked Severe Combined Immunodeficiency (Abstract Number: 608)
Oral presentation: Harry Malech, M.D., Laboratory of Clinical Immunology and Microbiology, National Institute of Allergy and Infectious Diseases, National Institutes of Health (NIH), Bethesda, MD, USA

Early outcome data for five older children and young adults with XSCID who received the lentivector (also known as lentiviral) gene therapy as salvage therapy after having previously received haplo-identical hematopoietic stem cell transplantation (HSCT) as infants without

chemotherapy-based conditioning were previously reported and published in *Science Translational Medicine*. By 2016, three additional patients were treated, and the cohort of eight patients (referred to as Cohort A) has now been followed for 3 to 7 years. Among Cohort A, gradual clinical benefit in the clearance of chronic norovirus and associated improved abdominal complaints, malabsorption, growth and IgG production were observed, and four patients were able to cease immunoglobulin replacement therapy.

While the results were positive, the relatively inefficient transduction of hematopoietic stem/progenitor cells (HSPCs) required large quantities of vector. This resulted in relatively low VCN in myeloid cells in some patients, with delayed immune cell recovery and persistent clinical disease, especially in the last patient treated (patient 8). To address this, NIH developed a refined enhanced transduction (ET) procedure consisting of a single overnight transduction after 48 hours pre-stimulation in cytokines (Stem cell factor, Thrombopoietin, Flt3-ligand; 100ng/mL) and incorporated transduction enhancers LentiBoost 1:100 and dimethyl prostaglandin 2 (dmPGE2; 1uM).

The presentation at the 2019 ASH Annual Meeting included data from six patients (referred to as Cohort B) treated by NIH, including re-treatment of patient 8. The patients, who were aged 12 to 36, had significant problems with donor T cell infiltration of liver, bone marrow and kidneys, and were nearly absent of B and NK cells. The enhanced transduction procedure achieved much greater transduction efficiencies than were observed in Cohort A, with greater than 10-fold less vector, and resulted in faster immune reconstitution and more significant clinical benefit by 3 months.

“We are encouraged by the significantly improved measures of early clinical outcomes from lentivector gene therapy in older children and young adults with XSCID using an enhanced transduction procedure with the addition of LentiBoost and dmPGE2,” said Dr. Malech. “Notably, we have seen an early appearance of B and NK cells at much higher levels in Cohort B than we previously observed in Cohort A, even at years after treatment. We look forward to continuing to closely monitor patients and report outcomes.”

About Mustang Bio

Mustang Bio, Inc. (“Mustang”) 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 and CRISPR/Cas9-enhanced 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. 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.

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