April 17, 2019

St. Jude Children's Research Hospital publishes results of Lentiviral Gene Therapy for Treatment of Infants with X-linked Severe Combined Immunodeficiency in New England Journal of Medicine

Licensing partnership for vector between St. Jude and Mustang Bio announced in Summer, 2018

This Phase 1/2 trial in newly diagnosed infants with XSCID continues to enroll at St. Jude, UCSF Benioff Children's Hospital and Seattle Children's Hospital

NEW YORK, April 17, 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, today announced that the *New England Journal of Medicine* has published data from St. Jude Children's Research Hospital ("St. Jude"), the nation's leading hospital dedicated to understanding, treating and curing childhood cancer and other life-threatening diseases. The data comes from a Phase 1/2 clinical trial of a lentiviral gene therapy for the treatment of newly diagnosed infants under two years old with XSCID, also referred to as SCID-X1 and commonly known as bubble boy disease. Under a licensing agreement with St. Jude, Mustang will develop the lentiviral gene therapy for commercial use as MB-107.

The multi-center Phase 1/2 clinical trial is evaluating the safety and efficacy of a lentiviral vector to transfer a normal copy of the *IL2RG* gene to bone marrow stem cells in newly diagnosed infants under the age of two with XSCID, preceded by low exposure-targeted busulfan conditioning. A total of 10 infants have received the therapy to date in this clinical trial. The published data covers eight infants with XSCID who were treated at St. Jude and at UCSF Benioff Children's Hospital and followed for a median of 16.4 months.

Data Highlights:

- Bone marrow harvest, busulfan conditioning and cell infusion were well tolerated.

- In seven of the eight cases, normalization of CD3+, CD4+ and CD4+ naïve T-cell and natural killer ("NK") cell numbers occurred within three to four months after treatment, accompanied by vector marking in T, B, NK and myeloid cells and marrow progenitors.
    - The eighth infant had insufficient T cells initially, but normalization of T cells occurred following an unconditioned boost of gene-corrected cells, and the patient is progressing favorably.
- All patients cleared previous infections and are growing normally.
- Seven of the eight infants treated have developed normal IgM levels to date.
    - Four of these seven infants have discontinued monthly infusions of intravenous immunoglobulin (IVIG) therapy to date.
    - Three of those four infants that discontinued monthly IVIG infusions have responded to vaccines to date.
- Most patients were discharged from the hospital within one month.

The gene therapy was developed in the laboratory of the late Brian Sorrentino, M.D., formerly of the St. Jude Department of Hematology, and produced in the St. Jude Good Manufacturing Practice facility. The multi-center Phase 1/2 clinical trial is led by Ewelina Mamcarz, M.D., assistant member at St. Jude. Other participating sites and key collaborators are at UCSF Benioff Children's Hospital, the National Institutes of Health (NIH) and Seattle Children's Hospital.

"The results have been very good thus far. We've been able to restore a full immune system pretty quickly," Mamcarz said. "All of these patients were able to come off of isolation and they've returned home with immune systems that were fully functional. We had patients come to us with very severe infections and they cleared them through the emergence of this newly developed immune system."

Martina Sersch, M.D., Ph.D., Chief Medical Officer of Mustang, said, "We are extremely encouraged by the Phase 1/2 clinical data published in the *New England Journal of Medicine*. They underscore the potential of MB-107 as a novel approach and potentially curative treatment option for newly diagnosed infants with XSCID. We are excited to continue working with St. Jude to evaluate MB-107 in this clinical trial, and we look forward to transferring the IND to Mustang by the end of this year, after which patients' cells from all three participating clinical trial sites will be processed in our Worcester, Mass., facility."

Manuel Litchman, M.D., President and Chief Executive Officer of Mustang, added, "In addition to the newly diagnosed infants with XSCID, we believe there are hundreds of patients with the disease who have been treated with hematopoietic stem cell transplantation, but who are experiencing decreasing T cell immunity and increasing incidence of infections. Through our collaborations with St. Jude and NIH, we are working to offer this life-saving therapy to these patients as well."

For additional information, visit ClinicalTrials.gov: NCT01512888.

About X-linked Severe Combined Immunodeficiency (XSCID)
X-linked severe combined immunodeficiency (XSCID) is a rare genetic disorder that occurs in approximately 1 per 200,000 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, also known as bubble boy disease, 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*; since *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. ("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.

St. Jude Children's Research Hospital

St. Jude Children's Research Hospital is leading the way the world understands, treats and cures childhood cancer and other life-threatening diseases. It is the only National Cancer Institute-designated Comprehensive Cancer Center devoted solely to children. Treatments developed at St. Jude have helped push the overall childhood cancer survival rate from 20 percent to 80 percent since the hospital opened more than 50 years ago. St. Jude freely shares the breakthroughs it makes, and every child saved at St. Jude means doctors and scientists worldwide can use that knowledge to save thousands more children. Families never receive a bill from St. Jude for treatment, travel, housing and food - because all a family should worry about is helping their child live. To learn more, visit stjude.org or follow St. Jude on social media at @stjuderesearch.

About Fortress Biotech

Fortress Biotech, Inc. ("Fortress") is an innovative biopharmaceutical company focused on identifying, in-licensing and developing high-potential marketed and development-stage drugs and drug candidates. The company has over 25 programs in development at Fortress, at its majority-owned and majority-controlled partners and at partners it founded and in which it holds significant minority ownership positions. Such product candidates span six large-market therapeutic areas, including oncology, rare diseases and gene therapy, which allow it to create value while mitigating risk for shareholders. Fortress advances its diversified pipeline through a streamlined operating structure that fosters efficient drug development. The Fortress model is driven by a world-class business development team that is focused on leveraging its significant biopharmaceutical industry expertise to further expand the company's portfolio of product opportunities. Fortress has established partnerships with some of the world's leading academic research institutions and biopharmaceutical

companies to maximize each opportunity to its full potential, including Alexion Pharmaceuticals, Inc., City of Hope, Fred Hutchinson Cancer Research Center, InvaGen Pharmaceuticals, Inc. (a subsidiary of Cipla Limited), St. Jude Children's Research Hospital and UCL Business PLC. For more information, visit www.fortressbiotech.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.

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

Investor Relations Contact:
Daniel Ferry
LifeSci Advisors, LLC
(617) 535-7746
daniel@lifesciadvisors.com

Media Relations Contact:
Tony Plohoros
6 Degrees
(908) 940-0135
tplohoros@6degreespr.com

MUSTANGBIO

Source: Mustang Bio, Inc.