



Mustang Bio and St. Jude Children's Research Hospital announce MB-107 lentiviral gene therapy for X-linked Severe Combined Immunodeficiency (XSCID) granted regenerative medicine advanced therapy (RMAT) designation from FDA

RMAT designation follows positive Phase 1/2 clinical data published in the New England Journal of Medicine

Designation to facilitate expedited development and review of MB-107 for XSCID

New York City and Memphis, Tennessee – August 22, 2019 – Mustang Bio, Inc. (NASDAQ: MPIO) and St. Jude Children's Research Hospital have announced that MB-107, a lentiviral gene therapy for the treatment of X-linked severe combined immunodeficiency, also known as bubble boy disease, has been granted the Regenerative Medicine Advanced Therapy (RMAT) designation by the U.S. Food and Drug Administration (FDA).

Mustang, 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, and St. Jude, the nation's leading hospital dedicated to understanding, treating and curing childhood cancer and other life-threatening diseases, established a partnership to continue development of the lentiviral gene therapy in August 2018.

Under the terms of the RMAT designation, the FDA will help facilitate the program's expedited development and review and provide guidance on generating the evidence needed to support the approval of MB-107 for XSCID. RMAT designation was granted to MB-107 based on positive Phase 1/2 clinical data for infants with XSCID under the age of 2 (ClinicalTrials.gov Identifier: [NCT01512888](https://clinicaltrials.gov/ct2/show/study/NCT01512888)), which were published in the [New England Journal of Medicine](https://doi.org/10.1056/NEJMoa1901001) in April 2019.

"We are very pleased that the FDA has granted RMAT designation to MB-107," said Manuel Litchman, M.D., president and chief executive officer of Mustang. "This exciting milestone further validates the compelling data generated to date and affirms the potential of MB-107 to address a typically fatal condition with few effective treatment options. We look forward to working with the FDA and our partners at St. Jude to potentially expedite the development and approval of this critically needed treatment option. In particular, we are on track to accept the Investigational New Drug (IND) transfer from St. Jude to Mustang in the fourth quarter of this year."

Drugs eligible for RMAT designation are those intended to treat, modify, reverse or cure a serious or life-threatening disease or condition, and that present preliminary clinical evidence indicating the drug has the potential to address unmet medical needs for such disease or condition. The FDA has determined that MB-107 for the treatment of XSCID meets the criteria for RMAT designation.

"Receiving RMAT designation by the FDA is an important step forward and should speed up the process of getting patients access to lentiviral gene therapy for XSCID," said Stephen Gottschalk, M.D., chair of the St. Jude Department of Bone Marrow Transplantation and Cellular Therapy. "We are excited to continue our collaboration with Mustang Bio and our academic partners to advance this new, potentially life-saving treatment option toward FDA approval."

RMAT designation provides regenerative medicine advanced therapy products with the same benefits to expedite the development and review of a marketing application that are available to drugs that receive Breakthrough Therapy Designation. These advantages include timely advice and interactive communications with FDA, as well as proactive and collaborative involvement by senior FDA managers and experienced review and regulatory health project management

staff. A product designated as an RMAT also may be eligible for other FDA-expedited programs, such as Priority Review. The FDA also may conduct a rolling review of products in its expedited programs, reviewing portions of a marketing application before the complete application is submitted.

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.

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 out license 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.

About 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% to 80% 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 www.stjude.org or follow St. Jude on social media at @stjuderesearch.

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 Mustang Bio's 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

St. Jude Media Relations Contacts:

Marvin Stockwell
Desk: (901) 595-6384
Cell: (901) 734-8766
marvin.stockwell@stjude.org
media@stjude.org

Michael Sheffield
Desk: (901) 595-0221
Cell: (901) 275-9065
michael.sheffield@stjude.org
media@stjude.org