



Mustang Bio and St. Jude Children's Research Hospital Enter into Exclusive Worldwide License Agreement for a Ground-Breaking, Clinical-Stage Lentiviral Gene Therapy with Curative Potential for X-linked Severe Combined Immunodeficiency

Mustang Expands Pipeline into Gene Therapy for Rare Disease

New York, NY and Memphis, TN – August 13, 2018 – Mustang Bio, Inc. (“Mustang”) (NASDAQ: MBIO), a Fortress Biotech (NASDAQ: FBIO) Company focused on the development of novel immunotherapies based on proprietary chimeric antigen receptor engineered T cell (CAR T) technology, and 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, announced today that they have partnered and entered into an exclusive worldwide license agreement for the development of a first-in-class *ex vivo* lentiviral gene therapy for the treatment of X-linked severe combined immunodeficiency (“X-SCID”), also known as bubble boy disease. X-SCID is the most common form of severe combined immunodeficiency, affecting approximately one in 50,000 to 100,000 newborns worldwide.

The therapy, which includes a low dose of busulfan prior to reinfusion of the patients' own gene-modified blood stem cells, is currently being evaluated in a Phase 1/2 multicenter trial in infants under the age of two at St. Jude, UCSF Benioff Children's Hospital San Francisco; and Seattle Children's Hospital. This study, led by Ewelina Mamcarz, M.D., Assistant Member at St. Jude, is the world's first lentiviral gene therapy trial for infants with X-SCID. In addition, the therapy is being investigated in patients over the age of two in a second Phase 1/2 trial at the National Institutes of Health (“NIH”). The therapy was developed in the laboratory of Brian Sorrentino, M.D., Director of the Division of Experimental Hematology at St. Jude.

Eight patients under the age of two with X-SCID have been treated to date, with results presented at the 21st Annual Meeting of the American Society of Gene & Cell Therapy in May 2018. The therapy was well tolerated. In addition, six patients achieved reconstituted immune systems within three to four months following treatment, with the remaining two patients continuing to progress favorably in earlier stages of recovery. Two of these six patients have discontinued monthly infusions of intravenous immunoglobulin, and the remaining patients, at earlier stages of recovery, continue to progress favorably. In three patients who had disseminated infections prior to therapy, all infections resolved completely.

“Our therapy has been well tolerated thus far, and none of the infants required any blood product support after low dose of busulfan,” Dr. Mamcarz said. “Most importantly, we observe recovery of all cells of the immune system, which is truly an achievement over prior gene therapy trials, where B cell reconstitution did not occur, and patients required intravenous immunoglobulin for life.”

Mustang and St. Jude believe there may be as many as 1,000 to 1,500 patients in the U.S. with X-SCID, as well as a similar number in Europe, who continue to have significant impairment of immunity despite receiving previous allogeneic stem cell transplantation and who therefore could be eligible for this gene therapy. In the NIH study, which was reported in the April 2016 issue of *Science Translational Medicine*, five patients aged 10 to 23 years with progressively declining persistent immune dysfunction after haploidentical hematopoietic stem cell transplant in infancy were treated with this *ex vivo* lentiviral gene therapy. The therapy appeared to be safe, and follow-up data from two older patients demonstrated immune system reconstitution and clinical improvement at 2 to 3 years following treatment. In three younger patients, similar levels of gene-modified immune cells were also observed at 6 to 9 months following treatment.

“X-SCID is a devastating genetic disorder of the immune system that occurs in infant males who, without treatment, do not live beyond infancy,” said Dr. Sorrentino. “We have seen compelling clinical data in which this X-SCID gene therapy enabled immune system reconstitution and the resolution of disseminated infections. We look forward to working with Mustang Bio to continue to advance the clinical development of this promising and potentially first-in-class gene therapy.”

Manuel Litchman, M.D., President and Chief Executive Officer of Mustang, said, “We are thrilled to announce the expansion of our pipeline into gene therapy for patients with X-SCID, a natural fit for our Worcester, Mass. cell processing facility. We look forward to working with St. Jude to advance this program through ongoing Phase 1/2 trials, with the goal of providing a novel, long-term treatment to the more than 80 percent of infants who lack fully matched bone marrow transplant donors and those patients who continue to have significant impairment of immunity. With our team’s extensive expertise in viral vector design, manufacturing and transduction, we are building a fully integrated cell and gene therapy company, with the goal of leveraging the transformative potential of these technologies to bring life-saving treatments to patients in need.”

About Mustang Bio

Mustang Bio, Inc. (“Mustang”), a Fortress Biotech Company, is a clinical-stage biopharmaceutical company focused on the development and commercialization of a broad range of proprietary chimeric antigen receptor engineered T cell (CAR T) immunotherapies and gene therapies in areas of unmet need. 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 lentiviral gene therapy for X-SCID. Mustang is registered under the Securities Exchange Act of 1934, as amended, and files periodic reports with the U.S. Securities and Exchange Commission. For more information, visit www.mustangbio.com.

About Fortress Biotech

Fortress Biotech, Inc. (“Fortress”) is a biopharmaceutical company dedicated to acquiring, developing and commercializing novel pharmaceutical and biotechnology products. Fortress develops and commercializes products both within Fortress and through certain of its subsidiary companies, also known as Fortress Companies. In addition to its internal development programs, Fortress leverages its biopharmaceutical business expertise and drug development capabilities and provides funding and management services to help the Fortress Companies achieve their goals. Fortress and the Fortress Companies may seek licensing arrangements, acquisitions, partnerships, joint ventures and/or public and private financings to accelerate and provide additional funding to support their research and development programs. For more information, visit www.fortressbiotech.com.

About St. Jude Children’s Research Hospital

St. Jude Children’s Research Hospital (“St. Jude”) 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 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 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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