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Mustang Bio Announces First Patient Successfully Treated by Ex Vivo Lentiviral Gene Therapy to Treat RAG1 Severe Combined Immunodeficiency

WORCESTER, Mass., July 27, 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 the first patient successfully received LV-RAG1 *ex vivo* lentiviral gene therapy to treat recombinase-activating gene-1 (“RAG1”) severe combined immunodeficiency (“RAG1-SCID”), in an ongoing Phase 1/2 multicenter clinical trial taking place in Europe. LV-RAG1 is exclusively licensed by Mustang for the development of MB-110, a first-in-class *ex vivo* lentiviral gene therapy for the treatment of RAG1-SCID.

Patients with SCID have mutations in blood stem cell genes that are responsible for the development and function of infection-fighting immune cells. As a result, they are unable to mount a normal defense response against infections. The administration of LV-RAG1 includes reduced intensity conditioning prior to reinfusion of the patients’ own gene-modified blood stem cells.

“The patient was administered LV-RAG1 without any complications. LV-RAG1 allowed the patient’s body to create a functioning immune system, which is responding well to the standard vaccinations for newborns,” said Arjan Lankester, Principal Investigator and Professor of Pediatrics and Stem Cell Transplantation at Leiden University Medical Centre (“LUMC”).

Manuel Litchman, M.D., President and Chief Executive Officer of Mustang said, “This first successful administration to a RAG1-SCID patient of a stem-cell based gene therapy represents a significant positive step forward for our MB-110 development program. This treatment, along with our X-linked severe combined immunodeficiency (“XSCID”) programs, which includes MB-107 and MB-207, has established Mustang as a leader in developing treatments for SCID patients, who are in great need of these life-saving therapies. XSCID and RAG1-SCID make up almost 60% of all SCID cases combined.[1] We look forward to continuing to advance these clinical candidates, including plans to initiate a multicenter pivotal Phase 2 trial for MB-107 under Mustang’s IND in the second half of this year.”

LV-RAG1 has been granted Orphan Drug Designation by the European Medicines Agency. Additional clinical trial sites are expected to be added in the near future.

Signed in 2021, Mustang's exclusive, worldwide license agreement for LV-RAG1 established an ongoing partnership with LUMC and LUMC's Frank J. Staal, Ph.D., molecular immunologist and professor of Molecular Stem Cell Biology. The license agreement grants Mustang rights to certain additional lentiviral gene therapies being developed in Dr. Staal's lab.

About RAG1-SCID

Severe combined immunodeficiency (SCID) due to complete RAG1 deficiency is a rare, genetic severe combined immunodeficiency disorder caused by null mutations in the *RAG1* gene resulting in less than 1% of wild type V(D)J recombination activity. Patients present with neonatal onset of life-threatening, severe, recurrent infections by opportunistic fungal, viral and bacterial micro-organisms, as well as skin rashes, chronic diarrhea, failure to thrive and fever. Immunologic observations include profound T- and B-cell lymphopenia, low or absent serum immunoglobulins, and normal natural killer cell counts. As is the case with other types of SCID, RAG1-SCID is fatal in infancy unless immune reconstitution is achieved with allogeneic hematopoietic stem cell transplantation ("HSCT"), or autologous stem cells corrected by gene therapy.

About MB-110 (*Ex Vivo* Lentiviral Gene Therapy)

MB-110 is a first-in-class *ex vivo* lentiviral gene therapy under development to treat RAG1-SCID, utilizing the LV-RAG1 vector developed in the laboratory of Frank J. Staal, Ph.D., molecular immunologist and professor of Molecular Stem Cell Biology at LUMC. Exclusively licensed to Mustang in 2021, LV-RAG1 is currently being evaluated in a Phase 1/2 multicenter, academic clinical trial (RECOMB) in Europe. Additional information on the trial can be found at http://www.clinicaltrials.gov using the identifier NCT04797260.

The same lentiviral vector drug substance produced by LUMC will be used to transduce patients' cells to create the MB-110 drug product produced at Mustang Bio's Worcester, MA, cell processing facility for further clinical development and to facilitate eventual commercial launch of the product.

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 candidates, 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. 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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[1] Fischer A, et al. *Nat Rev Dis Primers.* 2015; article number 15061; doi: 10.1038/nrdp.2015.61

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