

November 10, 2021



# **Mustang Bio Announces Exclusive Worldwide License Agreement with Leiden University Medical Centre for Clinical-Stage Lentiviral Gene Therapy with Curative Potential for RAG1 Severe Combined Immunodeficiency**

***Agreement expands Mustang's pipeline of lentiviral gene therapies for SCID***

***Preclinical development of additional targets to continue at Leiden University***

WORCESTER, Mass., Nov. 10, 2021 (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 company has executed an exclusive license agreement with Leiden University Medical Centre ("LUMC") for a first-in-class *ex vivo* lentiviral gene therapy for the treatment of RAG1 severe combined immunodeficiency ("RAG1-SCID").

The therapy, which includes low-dose conditioning prior to reinfusion of the patients' own gene-modified blood stem cells, is currently being evaluated in a Phase 1/2 multicenter clinical trial in Europe. The ongoing clinical trial recently enrolled its first patient, and additional clinical sites are expected to be added in the near future. The RAG1-SCID program has been granted Orphan Drug Designation by the European Medicines Agency.

Mustang also established an ongoing partnership with Frank J. Staal, Ph.D., professor of Molecular Stem Cell Biology and molecular immunologist, whose laboratory developed the therapy. Dr. Staal will continue the development of additional lentiviral gene therapies in his lab, to which Mustang Bio has rights under the agreement.

The RAG1-SCID therapy expands the pipeline of *ex vivo* lentiviral gene therapies currently in development at Mustang. The Company's lead programs, MB-107 and MB-207, are being investigated for the treatment of X-linked severe combined immunodeficiency ("XSCID"). A pivotal multicenter trial studying MB-107 is expected to enroll its first patient in the first quarter of 2022. XSCID and RAG1-SCID make up almost 60% of all SCID cases<sup>1</sup> combined.

Manuel Litchman, M.D., President and Chief Executive Officer of Mustang said, “We are excited to add RAG1-SCID to the Mustang portfolio as it enables us to leverage our lentiviral gene therapy expertise and experience and our state-of-the-art cell processing facility. Mustang is establishing itself as the leader in developing treatments for patients with severe combined immunodeficiency, an area of high unmet need. We have made great progress in moving our XSCID therapy into a registrational trial and look forward to similarly advancing this RAG1-SCID therapy to make it available for patients in need of life-saving treatment.”

### **About RAG1-SCID**

Severe combined immunodeficiency (SCID) due to complete recombinase-activating gene-1 (*RAG1*) deficiency is a rare, genetic severe combined immunodeficiency disorder due to 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 hematopoietic stem cell transplantation (HSCT).

### **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 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](http://www.mustangbio.com).

### **About Leiden University Medical Centre (“LUMC”)**

LUMC is a modern university medical center for research, education and patient care with a high quality profile and a strong scientific orientation. Its unique research practice, ranging from pure fundamental medical research to applied clinical research, places LUMC among the world top. Researchers at LUMC work together around 10 Research Themes: Academic Pharma, Neuroscience, Cell Tissue & Organ (Tx), Medical Genomics, Prevention & Lifestyle, Lifecourse Epidemiology and Geroscience, Immunity, Cancer, Cardio-Vascular, Infection.

LUMC strongly underpins the idea that ‘Science is the driving force behind innovative healthcare’. Outcomes of top level fundamental, translational and clinical research by LUMC researchers form a strong basis for innovative and qualitative healthcare on a national, European and international level.

### **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, and we claim the protection of the safe harbor for forward-looking statements contained in the Private Securities Litigation Reform Act of 1995.

**Company Contacts:**

Jaclyn Jaffe and Bill Begien  
Mustang Bio, Inc.  
(781) 652-4500  
[ir@mustangbio.com](mailto:ir@mustangbio.com)

**Investor Relations Contact:**

Daniel Ferry  
LifeSci Advisors, LLC  
(617) 430-7576  
[daniel@lifesciadvisors.com](mailto:daniel@lifesciadvisors.com)

**Media Relations Contact:**

Tony Plohoros  
6 Degrees  
(908) 591-2839  
[tplohoros@6degreespr.com](mailto:tplohoros@6degreespr.com)

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



Source: Mustang Bio, Inc.