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Fate Therapeutics Presents Efficacy Data for WNT7a-Analog Program at Muscular Dystrophy Association 2013 Scientific Conference

San Diego, CA – Fate Therapeutics, Inc., a biopharmaceutical company engaged in the discovery and development of adult stem cell modulators, announced today the presentation of preclinical data from its WNT7a protein analog program for the treatment of muscular dystrophy at the Muscular Dystrophy Association (MDA) 2013 Scientific Conference, April 21-24, in Washington DC. The presentations describe the engineering of pharmaceutically optimized WNT7a protein analogs, as well as their mechanism of action and efficacy profile in preclinical pharmacology studies.

“The data presented today provide strong preclinical support for the therapeutic potential of WNT7a analogs in muscular dystrophy, a complex group of disorders with a large unmet need for novel, differentiated and potentially complementary treatment approaches,” commented Christian Weyer, M.D., M.A.S., President and Chief Executive Officer of Fate Therapeutics. “We are working towards the nomination of an investigational new drug (IND) candidate later this year, and are excited about the potential to advance this new mechanism toward clinical studies.”

In the MDX mouse model of muscular dystrophy, intramuscular injection of a novel WNT7a analog resulted in significant dose dependent muscle hypertrophy and several-fold expansion of the satellite stem cell population. Moreover, three weeks after a single intramuscular injection, functional assessment revealed a significant increase in strength of the targeted tibialis anterior muscle (+18%, $p < 0.001$). These functional improvements were accompanied by a significant reduction in markers of fiber necrosis and inflammation typically seen in dystrophic muscle. Significant muscle hypertrophy and satellite stem cell expansion were also observed in wild-type mice. The WNT7a analogs assessed in these studies were engineered to overcome several key challenges of Wnt-family protein manufacture and formulation while retaining full biological activity and specificity. These and other data suggest the potential of WNT7a-based protein therapeutics across a range of neuromuscular diseases and conditions.

The findings obtained with Fate’s optimized WNT7a analogs expand upon those previously reported with non-modified WNT7a protein. In November 2012, muscle biology expert and Fate Therapeutics scientific founder Dr. Michael Rudnicki published data demonstrating the potential of WNT7a to ameliorate muscle degeneration in the MDX mouse model of muscular dystrophy (Von Maltzahn et. al., PNAS 2012). In previous studies, Dr. Rudnicki elucidated the unique biology of WNT7a and its dual mechanism of action of driving the expansion of the muscle satellite stem cell population and muscle hypertrophy.

About Muscular Dystrophy

Muscular dystrophies encompass a group of disorders with diverse pathophysiological manifestations resulting from genetic aberrations which include mutations or deletions to over 30 distinct genes. The most prevalent and well characterized is Duchenne muscular dystrophy (DMD), an X-linked form of muscular dystrophy which is seen in 1/3500 live male births. DMD typically manifests in early childhood and progresses to an advanced stage of severe muscular degeneration resulting in impairment of ambulation and premature mortality. A core pathophysiological phenomenon seen in muscular dystrophy is a cycle of muscle degeneration leading to continuous compensatory satellite cell activation and differentiation to affect a regenerative response, but resulting in the eventual exhaustion of the regenerative capacity and significant loss of muscle function. Enhancing the underlying molecular and cellular mechanisms to restore the regenerative capacity of muscle satellite stem cells thus represents a promising and unique approach for therapeutically intervention in various forms of muscular dystrophy as well as other neuromuscular diseases.

About Fate Therapeutics, Inc.

Uniquely positioned at the intersection of stem cell science and orphan disease, Fate Therapeutics is pioneering the discovery and development of innovative adult stem cell modulator therapeutics with the potential to cure or transform the lives of patients with rare life-threatening disorders. The Company’s lead program, ProHema, an innovative cord blood-derived cell therapy containing ex-vivo pharmacologically-modulated hematopoietic stem cells (HSCs), is currently in Phase 2 testing in patients with leukemia undergoing hematopoietic transplantation. The Company plans to pursue clinical evaluation of pharmacologically modulated HSCs in patients with rare genetic disorders, an area of tremendous unmet medical need in which the curative potential of cord blood transplantation is well recognized. In addition, Fate Therapeutics is developing proprietary WNT7a-based protein therapeutics that have shown efficacy in preclinical models of muscular dystrophy. To advance its discovery efforts, the Company applies its award-winning, proprietary, induced pluripotent stem cell (iPSC) technology to generate rare cell populations and model disease. Fate Therapeutics is headquartered in San Diego, CA, with a subsidiary in Ottawa, Canada. For more information, please visit www.fatetherapeutics.com.

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