Myonexus Therapeutics, Inc. Launches to Develop Pioneering, Clinical-Stage Gene Therapies Targeting Limb-Girdle Muscular Dystrophies

by Myonexus Therapeutics, Inc. | 5 June 2017 | Press Release |

- Innovative Gene Therapies with Potential to Become First-ever, Standard of Care Therapies for Limb-Girdle Muscular Dystrophies
- Announces Management Team of Industry Leaders with Technical and Commercial Expertise in Muscular Dystrophy Gene Therapy, Pharmaceuticals, and New Venture Development
- Licenses Innovative Gene Therapy Technologies from Nationwide Children's Hospital, Columbus, Ohio
- Secures Initial Financing from Global Limb-Girdle Muscular Dystrophy Community

New Albany, OH, 5 June 2017 – **Myonexus Therapeutics, Inc. (Myonexus)**, a clinical-stage biotechnology company developing transformative gene therapies for limb-girdle muscular dystrophies (LGMDs), today announced its launch plans. The company is committed to accelerating development of five experimental gene therapies pioneered within the laboratories of Louise Rodino-Klapac, Ph.D. and Jerry Mendell, M.D., Principal Investigators at the Research Institute at Nationwide Children's Hospital Center for Gene Therapy in Columbus, Ohio, USA.

Myonexus Therapeutics' pipeline includes Phase I programs for LGMD2D (α-sarcoglycanopathy) and LGMD2B (dysferlinopathy). Additionally, the company will advance its LGMD2E (β-sarcoglycanopathy) program into the clinic by late 2017 and simultaneously will advance two preclinical programs targeting LGMD2C (γ-sarcoglycanopathy) and LGMD2L (anoctamin 5). The Research Institute at Nationwide Children's will manufacture Phase I clinical supplies and will be the site of first in-human Phase I clinical trials.

"Myonexus Therapeutics' highly innovative experimental gene therapies offer potentially transformative quality of life improvements for limb-girdle muscular dystrophy patients," said Michael Triplett, Ph.D., Myonexus' President and Chief Executive Officer. "We are committed to advancing the trailblazing work of Dr. Rodino-Klapac, Dr. Mendell, and the entire Research Institute at Nationwide Children's Hospital Center for Gene Therapy team."

Joining Dr. Triplett on the leadership team are Bruce Halpryn, Ph.D., Chief Operating Officer, and Dr. Rodino-Klapac, Chief Scientific Officer. Providing expertise in specialty pharmaceuticals, drug delivery innovation and new ventures, Dr. Triplett previously served as Chief Executive Officer of N8 Medical, Inc. and as an executive at Battelle. Dr. Halpryn previously led early phase R&D, EU regulatory affairs, and technology scouting for Procter & Gamble Pharmaceuticals, overseeing development of numerous successful drug development programs. Dr. Rodino-Klapac is a recognized leader in LGMD and Duchenne muscular dystrophy gene therapy discovery and development. Dr. Mendell, a recognized global leader in clinical gene therapy and muscular dystrophy therapeutic development, will serve as a clinical development consultant to Myonexus.

"Based on our strong preclinical and early clinical data, I am excited to accelerate clinical development of our LGMD gene therapy pipeline and eager to work with the Myonexus team to continue translating our neuromuscular disease gene therapy expertise into potentially approved medicines," said Dr. Rodino-Klapac.

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"In the Center for Gene Therapy we are excited to facilitate the launching of Myonexus focused on products showing promise in the laboratory. Our past performance of clinical success in neuromuscular gene therapy clinical trials combined with our world-class research gene therapy capabilities is consistent with and validates our long-term vision and strategy to position Nationwide Children's Hospital and Columbus, Ohio as a global leader in addressing clinical unmet needs associated with neuromuscular diseases," said Dr. Mendell.

The LGMD community is providing Myonexus with initial financing, scientific contributions, and patient engagement assistance. "The global LGMD community is excited to support Myonexus Therapeutics. We look forward to transforming the lives of LGMD patients throughout the world. As we have seen in recent cases, strong patient advocacy support is critical to the success of rare disease companies. I am thrilled to support the company and to join its Board of Directors," said Bryan Barber, co-Founder of Myonexus and President, LGMD2D Foundation.

About Myonexus Therapeutics

Myonexus Therapeutics is a clinical stage, rare disease gene therapy company developing first ever treatments for Limb-girdle muscular dystrophies (LGMDs) based on research at Nationwide Children's Hospital, a leader in neuromuscular gene therapy discovery and translational research. Myonexus Therapeutics' pipeline includes three clinical stage gene therapy programs (LGMD2E, LGMD2D, and LGMD2B) and two preclinical gene therapy programs (LGMD2C and LGMD2L). Founded in 2017, Myonexus is headquartered in New Albany, Ohio. More information is available at www.myonexustx.com.

About Nationwide Children's Hospital

Named to the Top 10 Honor Roll on U.S. News & World Report's 2016-17 list of "America's Best Children's Hospitals," Nationwide Children's Hospital is America's largest not-for-profit freestanding pediatric healthcare system providing wellness, preventive, diagnostic, treatment and rehabilitative care for infants, children and adolescents, as well as adult patients with congenital disease. Nationwide Children's has a staff of more than 11,000 providing state-of-the-art pediatric care during more than 1.2 million patient visits annually. As home to the Department of Pediatrics of The Ohio State University College of Medicine, Nationwide Children's physicians train the next generation of pediatricians and pediatric specialists. The Research Institute at Nationwide Children's Hospital is one of the Top 10 National Institutes of Health-funded freestanding pediatric research facilities. More information is available at NationwideChildrens.org.

About LGMD2D Foundation

The LGMD2D Foundation is a registered 501(c)3 non-profit foundation whose mission is to expedite the development of a cure or therapy for Limb-girdle Muscular Dystrophy 2D (LGMD2D). In addition to educating patients and physicians, the Foundation maintains a patient registry, funds and monitors research and progress, provides financial support to accelerate clinical trials, and encourages scientific collaboration.

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