MDA, CMTA Announce Grant for Collaborative Non-Viral Gene Therapy Research on CMT1X

GLENOLDEN, PA, USA, September 12, 2023 /EINPresswire.com/ -- The Muscular Dystrophy Association (MDA) and Charcot-Marie-Tooth Association (CMTA) today announced a collaborative research grant totaling $299,992 to test gene delivery to Schwann cells using nanoparticles to treat CMT1X.

The three-year study, entitled Nanoparticle-based Gene Delivery to Schwann Cells for Treating CMT Disease, will be led by Alexia Kagiava, MD, of the Cyprus Institute of Neurology & Genetics in Nicosia, Cyprus.

Positive results from this study will provide a novel strategy for gene delivery to the peripheral nervous system that is anticipated to be more targeted and safer for clinical translation than adeno-associated virus (AAV) gene delivery strategies. The novel Schwann-cell targeted nanoparticle delivery is also anticipated to apply to other forms of Charcot-Marie-Tooth disease (CMT) and other demyelinating neuropathies caused by gene defects in Schwann cells.

CMT1X is a common inherited neuropathy, characterized by progressive muscle atrophy, weakness and sensory loss in the limbs. It is caused by mutations affecting connexin32 (Cx32), a protein responsible for the formation of gap junction channels in the myelin sheath and plays an important role in nerve function and integrity. Although researchers have developed an effective gene therapy approach for treating CMT1X by intrathecal injection of the gene encoding Cx32 to mice using AAV delivery, potential long-term toxicity and lack of cell specificity may limit clinical translation.

To develop a safer and potentially more targeted approach, this study aims to design a novel aptamer-conjugated nanoparticle carrying the gene expressing Cx32 that would enable gene entry specifically to Schwann cells in the CMT1X mouse model. This targeted nanoparticle approach is anticipated to result in a more targeted biodistribution and provide a safer and more translatable gene therapy for CMT1X, as well as other demyelinating neuropathies caused by

gene defects in Schwann cells.

“This project applies the latest techniques in nanoparticle technology to tackle a major challenge in the development of CMT treatments, getting them to the part of the body where they are needed,” said Katherine Forsey, Ph.D., chief research officer, CMTA. “The goal of the CMTA’s Strategy to Accelerate Research (STAR) is to develop treatments for CMT and this exciting new project builds on our long-standing collaborative relationship with the MDA as we work together to achieve that goal.”

Sharon Hesterlee, Ph.D., Chief Research Officer, MDA said “We are thrilled to collaborate with CMTA in order to fund meaningful research that could impact many individuals living with Charcot-Marie-Tooth disease. Advancements in treatments and cures for CMT are a big focus of the Association and will help accelerate MDA's overall mission to improve the lives of neuromuscular disease patients."

CMT is a spectrum of nerve disorders that affects some 3 million people globally. It causes damage to the peripheral nerves, which carry signals and relay sensations such as pain and touch to the brain and spinal cord from the rest of the body. CMT is caused by defects in the genes that are responsible for creating and maintaining the myelin (insulating sheath around many nerves, increasing conductivity) and axonal structures. More than 100 CMT-causing gene mutations have been identified. The vast majority of cases are attributed to mutations in just four genes: PMP22 (CMT1A), MPZ (CMT1B), GJB1 (CMT1X), and MFN2 (CMT2A).

CMT can be inherited in several ways: autosomal dominant (through a faulty gene contributed by either parent); autosomal recessive (through a faulty gene contributed by each parent); or X-linked (through a gene on the X chromosome contributed by either parent).

The CMTA is the largest philanthropic funder of CMT research worldwide. The CMTA’s Strategy to Accelerate Research (STAR) brings the best CMT researchers, clinicians, and experts in therapy development together with patients, pharmaceutical and biotechnology companies to expedite the development of treatments for CMT. Since 2008, the CMTA has invested more than $23.5 million via STAR. The CMTA also improves quality of life for CMT families by offering educational programs and materials, patient and professional conferences, support through youth programs, its branch system and global clinical Centers of Excellence.

The MDA is the leading voluntary health organization in the United States for people living with muscular dystrophy, ALS, and related neuromuscular diseases. For over 70 years, MDA has led the way in accelerating research, advancing care, and advocating for the support. As the largest source of funding for neuromuscular disease research outside of the federal government, MDA has invested more than $1 billion collectively in the laboratories of 7,000 scientists, helping build the field of neuromuscular disease biology and pioneering technologies such as the identification of disease-causing genes, gene therapy, antisense oligonucleotides, and, most recently, gene editing.

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