CMT Research Foundation Invests in Project at Nationwide Children's Hospital to Improve Gene Therapy Delivery for CMT

ATLANTA, GA, USA, July 31, 2024 /EINPresswire.com/ -- The CMT Research Foundation, a non-profit focused solely on delivering treatments and cures for Charcot-Marie-Tooth disease (CMT)*, has invested in a research project to develop new vehicles for delivering gene therapies more efficiently into peripheral nerves. This will improve treatment efficacy and safety of gene therapies for several types of CMT, including the most prevalent form, CMT1A. The project is based at Nationwide Children's Hospital (Columbus, Ohio) under the direction of Afrooz Rashnonejad, PhD, a principal investigator in the Center for Gene Therapy in the Abigail Wexner Research Institute at Nationwide Children's.

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>
> *Cleary Simpson, CEO of the CMTRF*

"The development of safe and effective gene therapies for CMT requires the delivery of the therapeutic genetic payload into the peripheral nervous system, specifically targeting Schwann cells for certain CMT types, including but not limited to CMT1A, 1B, 4C, 4J, and 1X. Special carriers called adeno-associated viral (AAV) vectors currently serve as the major delivery method for gene therapies," says Dr. Rashnonejad. "One of the major barriers for developing an AAV-based gene therapy for CMT is getting sufficient delivery of the therapeutic payload to the peripheral nerve, specifically the Schwann cells within the nerve for demyelinating CMTs. Current studies on AAV delivery systems studies in mice have shown that only a portion of Schwann cells in the peripheral nerve are transduced. This is more challenging when you want to target Schwann cells in large animals including NHPs and humans. This project aims to develop new AAV-based vehicles for delivering gene therapies more efficiently into the Schwann cells."

Dr. Rashnonejad's collaborator on this project, Dr. Nerea Zabaleta, is an Assistant Professor at Harvard Medical School and a researcher at the Grousbeck Gene Therapy Center at Mass Eye and Ear, part of the Ocular Genomics Institute within Mass General Brigham.

“The team conducting the experiments possess extensive experience and expertise in AAV gene therapy development, with solid track records,” says Cleary Simpson, CEO of the CMTRF. “The delivery of therapies to peripheral nerves continues to pose a challenge in treating CMT. There is value in a study aimed at optimizing the capsid of AAVs to enhance targeting efficiency.”

The Abigail Wexner Research Institute (AWRI) is one of the top 10 National Institutes of Health-funded free-standing pediatric research facilities in the U.S., supporting basic, clinical, translational, behavioral, and population health research. The AWRI is comprised of multidisciplinary Centers of Emphasis paired with advanced infrastructure supporting capabilities such as technology commercialization for discoveries; gene- and cell-based therapies; and genome sequencing and analysis.

CMT Research Foundation (CMTRF) is a patient-led, non-profit focused on delivering treatments and cures for CMT. The foundation identifies significant obstacles or deficiencies impeding progress toward a cure and seeks out collaborators to address these issues. To date, CMTRF has funded 24 projects, of which 8 are completed. Of those 8 completed projects, 5 have clinical candidates. CMTRF's mission to invest in promising science with high potential of leading to treatments and cures was proven effective and ground-breaking when DTx Pharma with a CMTRF- backed program as its lead candidate was acquired by Novartis for $1 billion. Founded by two patients who are driven to expedite drug delivery to people who live with CMT, the 501(c)(3) federal tax-exempt organization is supported by personal and corporate financial gifts.

*Charcot-Marie-Tooth encompasses a group of inherited, chronic peripheral neuropathies that result in nerve degradation. CMT patients suffer from progressive muscle atrophy of legs and arms, causing walking, running and balance problems as well as abnormal functioning of hands and feet. CMT affects one in 2,500 people (about the same prevalence as cystic fibrosis), including 150,000 Americans and nearly 3 million people worldwide. At the moment, there is no treatment or cure for CMT.

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