CMTA's $240K Investment Targets Gene Replacement Therapy in CMT4 Subtypes

CMTA announces $240K investment in gene therapy for CMT4A, pioneering treatment with potential for other CMT types.

GLENOLDEN, PENNSYLVANIA, UNITED STATES, June 18, 2024 /EINPresswire.com/ -- CMTA's $240K Investment Targets Gene Replacement Therapy in CMT4 Subtypes

> Through the STAR initiative, CMTA accelerates gene replacement therapies, paving the way for clinical trials. Leveraging advanced AAV vector tech, we're creating hope for the broader CMT community."
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> *Dr. Katherine Forsey, CMTA Chief Research Officer*

The Charcot-Marie-Tooth Association (CMTA), the largest philanthropic funder of Charcot-Marie-Tooth (CMT) disease research aimed at bringing treatments to patients, announced today an investment of $240K into a new gene replacement therapy for CMT4A, with a potential to translate to other types of CMT through a templated approach. Xin Chen, MD, PhD, and CMTA Scientific Advisory Board member Steven Gray, PhD, will lead this important work at the University of Texas Southwestern Medical Center in Dallas, Texas. This investment is part of a key strategic imperative at CMTA to support research with broad impacts for patients.

Recessive GDAP1 gene variants cause CMT4A, a severe demyelinating form of CMT. This project will involve designing and testing an adeno-associated virus 9 (AAV9) vector. Drs. Chen and Gray aim to replace the faulty GDAP1 gene with a fully functional version. This approach will be tested in a model replicating the conditions of CMT4A, providing a crucial step towards potential treatment for this type of CMT. Should this early work prove successful, the researchers believe this approach could be used as a template for other types of CMT, such as CMT4B1 and CMT4D.

"Recent advances in developing desirable AAV capsids, optimizing genome designs, and harnessing modern biotechnologies have dramatically contributed to the growth of the AAV gene therapy field," said Dr. Chen. "With these constantly evolving and improving AAV vector technologies, we believe that studies with relatively rare forms of CMT, such as CMT4A, can provide proof-of-concept for rapidly developing CMT gene therapies. As better AAV vector technology becomes available, we can expand the reach of this 'templated' approach to more forms of CMT."

“Through the Strategy to Accelerate Research (STAR) initiative, CMTA has been at the forefront of accelerating gene replacement therapies for CMT, underscoring our leadership in this critical area of research,” said Katherine Forsey, PhD, CMTA Chief Research Officer. “This investment in gene replacement therapy represents a significant step towards developing a roadmap for rapidly translating treatments for CMT into clinical trials. By leveraging advanced AAV vector technologies, we are creating a blueprint for future therapies, bringing hope to the broader CMT community.”

About CMT

Named after the three doctors who first described it in 1886: Charcot, Marie, and Tooth, CMT affects one in every 2,500 people—a rare disease subdivided into multiple subtypes, each with a lower prevalence. Due to the degradation of their nerves, people with CMT suffer lifelong progressive muscle weakness and atrophy of the arms and legs, and can affect other parts of the body. This leads to problems with balance, walking, hand use, and more. There currently is no treatment or cure for this debilitating disease.

About CMTA

CMTA is a community-led, community-driven 501(c)(3) nonprofit organization with a mission to support the development of new treatments for CMT, to improve the quality of life for people with CMT, and, ultimately, to find a cure. As the leading global philanthropic funder of CMT research, CMTA unites the community with clinicians and industry experts to accelerate the advancement of treatments, with investments of more than $24 million since 2008. For more information, visit https://www.cmtausa.org

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