

CMTA Advances Drug Repurposing with an Additional \$225,720 Investment in CMT1B and CMT1E Research

Project extends ongoing CMTA-funded research in CMT1A that targets protein-clearance machinery to restore peripheral nerve function.

GLENOLDEN, PA, UNITED STATES, August 11, 2026 /EINPresswire.com/ -- The Charcot-Marie-Tooth Association (CMTA) announced a \$225,720 investment in new research led by Maurizio D'Antonio, PhD, at Ospedale San Raffaele in Milan, Italy. The study



tests two treatments for their ability to restore peripheral nerve function in CMT1B and CMT1E by enhancing the protein-clearance machinery in Schwann cells. The first is an FDA-approved phosphodiesterase type 5 (PDE5) inhibitor that raises cyclic GMP levels. The second is a therapeutic candidate from a Strategy to Accelerate Research (CMTA-STAR) Alliance Partner.

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*Katherine Forsey, PhD, CMTA
Chief Research Officer*

CMTA's commitment to drug repurposing is key to the organization's strategy to move therapeutic solutions through the drug development pipeline as quickly as possible. Because repurposed drugs already have established safety data, they can reach patients faster and at lower cost than a new molecule — a decisive advantage for a rare disease like CMT, which still has no approved treatments.

In CMT1B and CMT1E, gene mutations cause peripheral nerve myelin proteins to misfold and build up inside Schwann cells. This buildup stresses the cell's main system for clearing damaged proteins, the proteasome, and drives peripheral nerve degeneration. [Ongoing CMTA-funded research](#) led by Jordan VerPlank, PhD, is investigating this pathway in CMT1A and has shown that targeting it with a repurposed medicine restores proteasome activity and improves peripheral

nerve function in preclinical CMT1A models. The D'Antonio lab will test whether the same strategy applies to CMT1B and CMT1E, and potentially to other forms of CMT, advancing a key CMTA-STAR goal of targeting shared biological pathways across multiple CMT subtypes.

"We are grateful to the Charcot-Marie-Tooth Association for supporting this work, which addresses a critical gap in our understanding of how CMT1B and CMT1E damage the peripheral nerve," says D'Antonio. "This project is designed to define how disrupted protein handling contributes to peripheral nerve damage and to determine whether restoring this system could be developed into a treatment strategy for these subtypes and potentially others."



Maurizio D'Antonio, PhD

"The D'Antonio lab extends our CMT1A work into CMT1B and CMT1E and helps to determine whether this approach has broader application across multiple CMT subtypes," says Katherine Forsey, PhD, CMTA Chief Research Officer. "By connecting ongoing research from the VerPlank laboratory, repurposing approved drugs, and bringing in additional candidate therapeutics from CMTA-STAR Alliance Partners, this project demonstrates how CMTA-STAR unites academic researchers and industry around a shared biological approach that moves treatments to patients faster. If successful, this approach could help create new treatment options for people living with CMT."

About Charcot-Marie-Tooth Disease (CMT)

Charcot-Marie-Tooth disease is a group of rare, inheritable peripheral neuropathies. Named after Jean-Martin Charcot, Pierre Marie, and Howard Henry Tooth, who first described it in 1886, CMT causes progressive muscle weakness, loss of sensation, impaired balance, and other debilitating complications. There is currently no cure or approved treatment.

About the Charcot-Marie-Tooth Association (CMTA)

The Charcot-Marie-Tooth Association (CMTA) is the leading nonprofit organization dedicated to improving the lives of people living with Charcot-Marie-Tooth disease (CMT). As the largest

philanthropic funder of CMT research, CMTA has invested more than \$33 million since 2008 to accelerate research leading to new treatments and ultimately a cure. Through a strategic model that combines targeted funding, specialized scientific tools and resources, and strong patient partnership, CMTA drives promising science toward meaningful outcomes for people living with CMT. Learn more: cmtausa.org

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