

CMTA Announces \$165,000 Investment to Advance Treatments for CMT1A and CMT1E

New CMTA-funded research will study treatment timing, PMP22 activity and biomarkers to help guide future clinical trials for CMT1A and CMT1E.

GLENOLDEN, PA, UNITED STATES, September 8, 2026 /EINPresswire.com/ -- The Charcot-Marie-Tooth Association (CMTA) announced today a \$165,000 investment in a research project led by veteran researchers Michael Shy, MD, and Robert Piper, PhD, at the

University of Iowa to develop preclinical models to detect the optimal time for therapeutic intervention in CMT1A and CMT1E.



CMT1A and CMT1E are both linked to the PMP22 gene, which is essential for healthy peripheral nerve myelin (the protective sheath around the nerve). In CMT1A, an extra copy causes the body to make too much PMP22 protein, and in CMT1E, mutations damage peripheral nerve myelin early in life. This newly funded project will develop mouse models that assess the impact of PMP22 activity at different stages of disease progression. These models will also help identify biomarkers that signal whether therapy is working before functional changes can be measured. Understanding the

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*Katherine Forsey, PhD, CMTA
Chief Research Officer*

underlying biology of PMP22 will help to optimize drug treatments for these common CMT subtypes, a key research priority of CMTA's Strategy to Accelerate Research (CMTA-STAR).

“I see CMT patients every week who want to know when treatment will be ready and whether it will work,” says Dr. Shy. “Therapies targeting PMP22 are advancing, but clinical trials depend on understanding when treatment must begin, how much correction is needed, and how to measure whether nerves are responding. These models help answer those questions.”

“CMTA funds the research that makes clinical trials possible in CMT,” says Katherine Forsey, PhD,

CMTA Chief Research Officer. "Validated models and early biomarkers give researchers and industry partners the tools to design optimized clinical trials, evaluate therapies earlier in the disease, and understand when treatment is most likely to preserve nerve function. Every step toward better trials brings potential therapies closer to families living with CMT."

The work builds on a four-year collaboration between Drs. Shy and Piper. Their research has produced multiple peer-reviewed studies in CMT and myelin biology, including a 2025 [natural history study of CMT1E](#) published in Brain, which found that progression is fastest during childhood and adolescence, and linked PMP22 biology to disease severity.

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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