
Leading the Way: The CMTA Invests More Than \$200K in Cutting-Edge CMT1A Organoid Research

FOR IMMEDIATE RELEASE

- **Establishing standardized methods for generating organoid models of CMT**
- **Enabling scientists to efficiently evaluate potential treatments for CMT**

GLENOLDEN, PA, UNITED STATES, FEBRUARY 21, 2024—The Charcot-Marie-Tooth Association (CMTA), the largest philanthropic funder of Charcot-Marie-Tooth disease (CMT) research aimed at bringing treatments and a cure to patients, announced today an investment of more than \$200,000 in new funding to develop an innovative organoid model of CMT1A, the most common form of this disease. This cutting-edge approach will enable scientists to efficiently evaluate potential treatments that target defects in myelin, the protective sheath around peripheral nerves.

Led by Professor Vincent Timmerman, Ph.D., the Peripheral Neuropathy Research Group at the University of Antwerp in Belgium is spearheading a project aimed at assessing CMT1A organoids to accelerate drug testing. Dr. Timmerman is a pioneer in the creation of these miniature organs from induced pluripotent stem cells (iPSCs). “As we recently developed human-derived miniature organ-like structures, here named organoids,” said Dr. Timmerman, “we highly appreciate the support of the CMTA which will allow us to characterize this system for evaluating CMT1A neuropathy. The availability of a three-dimensional structure will allow a qualitative and quantitative assessment of candidate therapies enhancing preclinical studies.”

“As we embark on this pioneering project to develop organoid models for CMT1A, we’re not just addressing a singular aspect of the disease; rather, we’re contributing to a larger framework of research aimed at revolutionizing how we understand and treat CMT,” said Katherine Forsey, Ph.D., CMTA Chief Research Officer. “By investing in cutting-edge methodologies like organoid modeling, the CMTA is not only accelerating progress toward effective treatments for CMT1A but also laying the groundwork for advancements across multiple types of CMT. This strategic investment underscores our commitment to leveraging innovative approaches and collaborative partnerships to drive meaningful impact for CMT patients worldwide.”

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 the CMTA

The 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, the 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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