CMTA Awards Over Half a Million Dollars to Fuel Research in Demyelinating Forms of CMT

New 500K+ investment by the CMTA to find new treatments for CMT types 1A and 1B to solve an unmet need.

The Charcot-Marie-Tooth Association's Registered Logo

GLENOLDEN, PA, UNITED STATES, February 7, 2024 /EINPresswire.com/ -- The Charcot-Marie-Tooth Association (CMTA) announced today a groundbreaking international research initiative investing over $500,000 in new funding to cover two multiyear projects. The first, led by CMTA Scientific Advisory Board Chairperson, Professor John Svaren, Ph.D.., based at the University of Wisconsin, will explore novel pathways for lowering PMP22 expression in CMT1A using a small molecule approach. The second will see Professor Svaren working alongside CMTA scientific advisory board members Maurizio D'Antonio, Ph.D., at the San Raffaele Scientific Institute in Milan, Italy, and Bruce Conklin, M.D., at the Gladstone Institute in San Francisco, CA and lays the groundwork for new gene editing therapies for the most common demyelinating forms of Charcot-Marie-Tooth disease (CMT): CMT1A and CMT1B. This significant new investment demonstrates the CMTA's global leadership in advancing treatments for CMT and it underscores the association's commitment to improving the lives of those who have CMT, while strengthening its dedication to developing treatments and, ultimately, finding a cure.

The research focuses on two key aspects.

The first project aims to develop an orally administered drug that reduces the overexpression of PMP22, the root cause of CMT1A. Utilizing insights gained through previous CMTA funding, the Svaren laboratory at the University of Wisconsin seeks to normalize PMP22 levels in Schwann cells, improving disease symptoms and severity. If successful, the study will identify a novel agent for lowering PMP22 levels that could allow for rapid testing in human clinical trials in CMT1A.

In the second project, a gene editing approach utilizes CRISPR-Cas9 to design a universal approach for treating both CMT1A and CMT1B patients, regardless of their specific mutations. According to Dr. Svaren, "It is an exciting opportunity to explore the potential of genome editing in targeting CMT1A and CMT1B, anticipating optimal strategies for these common CMT types."

“Demyelinating CMTs affect over half of all people with this devastating disease. This latest investment through the CMTA’s Strategy to Accelerate Research (STAR) reflects our multi-modality approach to tackling CMT head-on. Through partnerships with world-leading research scientists and projects with rapid translational potential, we continue to accelerate progress towards the development of treatments,” said Katheriene Forsey, Ph.D., the CMTA’s Chief Research Officer.

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-focused, 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 the CMTA's website.

Kenny Raymond
Charcot-Marie-Tooth Association
+1 734-862-8702
email us here
Visit us on social media:
Facebook
Twitter
LinkedIn
Instagram
YouTube
TikTok

---

in today's world. Please see our Editorial Guidelines for more information.