CMTA's $300K Investment in CRISPR for CMT2A

CMTA invests $300K in CRISPR gene editing for CMT2A at UCSF's Gladstone Institutes, targeting MFN2 mutations to restore nerve function.

GLENOLDEN, PA, UNITED STATES, March 25, 2025 /EINPresswire.com/ -- The Charcot-Marie-Tooth Association (CMTA), the largest philanthropic funder of Charcot-Marie-Tooth disease (CMT) research, announced today a $300,000 investment in a cutting-edge gene editing therapy for CMT2A. Led by CMTA Strategy To Accelerate Research (CMTA-STAR) Advisory Board Member and Principal Investigator at Gladstone Institutes, Bruce Conklin, MD, this two-year project represents a critical step in CMTA's mission to bring transformative treatments to patients.

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> *Katherine Forsey, PhD, CMTA Chief Research Officer*

CMT2A, the most common axonal type of CMT, can be caused by over 200 different mutations in one copy of the MFN2 gene (mutant copy) while the other copy remains normal (healthy copy). Current research suggests that targeting and silencing only the mutant copy of MFN2 could restore peripheral nerve health. The Gladstone team's groundbreaking CRISPR/Cas9-based approach is designed to selectively silence the mutant MFN2 gene while preserving the healthy one. The Conklin lab is using CRISPR in a new innovative way to provide a single solution for many different mutations within a single gene, as opposed to having to 'fix' all 200 different mutations separately.

"Gene editing technologies like CRISPR/Cas9 offer exciting possibilities for treating CMT at its source," said Dr. Conklin. "This project provides a blueprint for therapeutic editing of most dominant forms of CMT, and our goal is to overcome critical technical barriers, including efficiently screening thousands of CRISPR designs and validating their effectiveness in human neurons."

The study will use identical human cell lines engineered to carry different CMT2A-causing mutations of the MFN2 gene. This controlled environment allows the Conklin Lab to rigorously test the safety and efficacy of CRISPR-based editing in restoring normal peripheral nerve function.

“This project underscores CMTA-STAR’s commitment to accelerate bold and impactful research,” said Katherine Forsey, PhD, CMTA Chief Research Officer. “By investing in these MFN2 gene editing strategies, we’re addressing the unmet needs of individuals with CMT2A while laying the foundation for therapies that could benefit many other CMT subtypes. The potential of this work to reshape treatment options underscores the power of innovative science.”

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. This rare disease has multiple subtypes, each with a lower prevalence. People with CMT experience progressive sensory loss, muscle weakness, and atrophy in the arms and legs, along with impaired balance, mobility, hand function, and more. There is currently 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 $30 million since 2008. For more information, visit cmtausa.org

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