# CMTA Invests $110,500 to Develop Novel ASO Medicine for CMT2A

*New CMTA partnership with La Jolla Labs will develop a first-of-its-kind ASO therapeutic targeting CMT2A, the most common axonal type of CMT.*

GLENOLDEN, PA, UNITED STATES, September 24, 2026 / EINPresswire.com/ -- The Charcot-Marie-Tooth Association (CMTA) announced today a $110,500 investment in a new partnership with La Jolla Labs to develop an antisense oligonucleotide (ASO) medicine targeting CMT2A, the most common axonal form of Charcot-Marie-Tooth disease (CMT). Targeted genetic therapies are an important objective of CMTA's research strategy because they address the underlying genetics of specific CMT subtypes, creating the potential for significant impact. This project will be accomplished in a unique public-private partnership, pairing the CMT expertise of CMTA Strategy to Accelerate Research (CMTA-STAR) Advisory Board Chair John Svaren, PhD, of the University of Wisconsin-Madison, with La Jolla Labs' specialized ASO development expertise.

> Targeted genetic approaches such as this are a key part of CMTA's research strategy. This new partnership with La Jolla Labs leverages the expertise needed to develop it."
>
> *Katherine Forsey, PhD, CMTA Chief Research Officer*

La Jolla Labs has previously collaborated with disease foundations, biotechnology companies, and academic institutions to advance RNA therapeutics for rare diseases, including personalized ASOs for nano-rare patients, exploration of ASO applications in Wolfram syndrome, and RNA-targeted therapeutic discovery for ALS.

For people living with CMT2A, progressive muscle weakness and loss of sensation lead to a loss in strength, mobility, and independence from a very early age, with no approved treatment. While underlying mutations in the MFN2 gene damage peripheral nerve neurons, published research shows that neurons affected by CMT2A have little to no natural expression of a related gene, MFN1. This project invests in an innovative approach to increase MFN1 expression in these

neurons to compensate for the damaging effects of MFN2 gene mutations.

“We are excited to apply our mission and expertise to CMT and look forward to fostering collaboration with the CMT research community to identify potential therapeutics for CMT2A patients,” said Tamar Grossman, CEO of La Jolla Labs.

“The published science tells us this strategy can work for CMT2A, and La Jolla Labs brings exactly the expertise needed to develop it,” said Katherine Forsey, PhD, CMTA Chief Research Officer. “Targeted genetic approaches such as this are a key part of CMTA’s research strategy. Our CMTA-STAR Advisory Board identified this as a strong approach for CMT2A, and if this unique partnership delivers results, we see a clear path to expanding ASO approaches across other subtypes.”

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 La Jolla Labs Inc.

La Jolla Labs Inc. is a clinical stage biotechnology company developing precision RNA therapeutics for rare and complex diseases. The company integrates RNA biology, artificial intelligence, computational design, and high-throughput screening to discover, optimize, and develop antisense oligonucleotides (ASOs) and other RNA-targeting modalities. Learn more: lajollalabs.ai

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 $38 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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