

# CMTA Invests \$281,339 to Advance Treatments for CMT1A

*CMTA invests \$281,339 in a new therapeutic pathway to accelerate treatments for CMT1A.*

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EINPresswire.com/ -- The Charcot-Marie-Tooth Association (CMTA), the largest philanthropic funder of Charcot-Marie-Tooth (CMT) disease research, announced today a \$281,339 investment in a three-year study to

explore an innovative therapeutic approach for CMT1A. The project, led by Sophie Belin, PhD, and Yannick Poitelon, PhD, at Albany Medical College, will evaluate whether a newly discovered regulatory pathway in Schwann cells (the key cells for myelination) can reduce PMP22 levels and restore nerve function.

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*Drs. Belin and Poitelon*

CMT1A is the most common form of CMT and is caused by an extra copy of the PMP22 gene, which leads to damage of the myelin sheath, the protective covering around peripheral nerves, and results in progressive weakness and sensory loss.

The newly funded study will test whether molecules originally in development as cancer therapeutics can also be used to lower PMP22 and improve nerve health in a

model of CMT1A. Since these molecules are already in development, this could accelerate the path for CMT if the approach proves successful.

“This CMTA Strategy To Accelerate Research (CMTA-STAR) project explores an encouraging approach to treating CMT by targeting a newly discovered pathway,” said Katherine Forsey, PhD, CMTA Chief Research Officer. “We are excited to break new ground in the development of treatments for CMT1A.”



“We’ve spent years in the lab studying Schwann cell biology and uncovering the mechanism behind peripheral neuropathies,” said Drs. Belin and Poitelon. “This project represents the pinnacle of that journey, finally reaching a point where our discoveries could translate into a treatment that targets the root cause of CMT1A.”

This latest investment reflects the CMTA-STAR commitment to advancing research that addresses the underlying biology of CMT. By supporting the development of a molecule in clinical testing for other diseases into CMT, CMTA is accelerating progress toward treatments.

#### 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



Sophie Belin, PhD, Albany Medical College



Yannick Poitelon, PhD, Albany Medical College

advancement of treatments, with investments of more than \$33 million since 2008. For more information, visit [cmtausa.org](https://cmtausa.org)

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