

CMTA | STAR Awards \$354,826 For Charcot-Marie-Tooth Clinical Trial

GLENOLDEN, PA, USA, December 7, 2021 /EINPresswire.com/ -- The Charcot-Marie-Tooth Association | Strategy to Accelerate Research (CMTA | STAR) awarded Dr. Mary M. Reilly and her team at the UCL Queen Square Institute of Neurology in London \$354,826 for a clinical trial in patients with Hereditary Sensory Neuropathy 1 (HSN1).



HSN, a type of Charcot-Marie-Tooth disease (CMT), is a rare autosomal dominant neuropathy that leads to severe sensory and motor impairment. The study will test the efficacy of L-serine in slowing or stopping disease progression in HSN1 secondary to SPTLC1/2 mutations.

Reilly's preliminary studies—together with previous preclinical trials in animals and an earlier pilot study in patients with HSN1—set the stage for a clinical trial of L-serine in patients with HSN1 secondary to SPTCL1/2 mutations. This will be the first clinical trial to use and validate MRI muscle fat fraction as a primary outcome measure in an inherited neuropathy. The findings will provide direct confirmation of its utility in phase II and phase III clinical trials for CMT and related neuropathies.

All of the CMTA's research efforts are consolidated under the banner of STAR—which brings together the world's largest network of biotech research partners, research scientists, clinicians and patients—and funds more CMT grants than any other philanthropic organization to increase the likelihood of finding a cure. Since 2008, the CMTA has invested more than \$17.5 million in STAR, with plans to invest another \$10 million in the next few years.

ABOUT CMT

CMT is a group of diseases caused by inherited genetic mutations that damage the peripheral nerves outside of the brain and spinal cord. Scientists have identified over 100 different gene mutations causing CMT. Most people (90 percent) have one of four types of CMT: CMT 1A (PMP 22); CMT 1B (MPZ); CMT 2A (MFN2) and CMT 1X (GJB1). It is estimated that CMT affects more than 3 million people worldwide, regardless of gender, race or ethnicity.

ABOUT THE CMTA

The CMTA is the largest philanthropic funder of CMT research worldwide. The CMTA's Strategy to Accelerate Research (STAR) brings the best CMT researchers, clinicians, and experts in therapy development together with pharmaceutical and biotechnology companies and patients to expedite the development of treatments for CMT. The CMTA is also actively working to help improve the quality of life for all families living with CMT by offering educational programs and materials, hosting patient and professional conferences, providing support to families through its nationwide branch system and more. More information can be found at <u>www.cmtausa.org</u>.

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