

## CMTA | STAR Funds CMT1A/CMT1B Project with “Considerable Translational Potential”

GLENOLDEN, PENNSYLVANIA, USA, April 6, 2021 /EINPresswire.com/ -- The Charcot-Marie-Tooth Association | Strategy to Accelerate Research (CMTA-STAR) announced a \$204,785 grant April 6 for a research project aimed at improving the proteasome function to treat both CMT Type 1A and 1B. Members of the CMTA's Scientific Advisory board said that based on very exciting preliminary data the project has considerable translational potential.

Dr. Laura Feltri from the University at Buffalo will helm the 12-month project, building on earlier work at the Feltri/Wrabetz laboratory to promote elimination of the mutant protein—myelin protein zero (P0). That work raised levels of the key signaling molecule cGMP to activate the proteasome, which is responsible for degradation of intracellular proteins.

Mutations in myelin protein zero, the predominant gene product of Schwann cells, cause CMT1B. Previous work has firmly established that the

accumulation of mutant MPZ protein in CMT1B causes activation of the unfolded protein response (UPR), leading to disease symptoms.

While other approaches to resolve the UPR are in testing (and clinical trials for CMT1A), drugs already approved for hypertension and erectile dysfunction are available for preventing



Dr. Laura Feltri

degradation.

Based on a successful pilot trial in a CMT1B mouse model, Feltri's research project will test two other drugs with more optimal pharmacology for treating the CMT1B S63del mouse model. The first aim will use short term studies of two drugs to establish optimal dosing, and the second aim will perform a longer efficacy trial. This approach will also be tested in a CMT1A model in parallel to see if these approved drugs could have some benefit for CMT1A. One company partner has been identified for these studies.

To read more about the CMTA's research, visit: <https://www.cmtausa.org/our-research/>

About CMTA | STAR:

CMTA | STAR is dedicated to accelerating research into the development of new drugs and treatments for CMT by funding more grants than any other philanthropic organization. CMTA | STAR brings together the world's largest network of biotech research partners, research scientists and clinicians to work with the patient community, increasing the likelihood of finding a cure.

In addition to driving research forward, the CMTA provides education, resources and support to patients via print publications, social media platforms and virtual meetings.

The CMTA's research and community services have earned it Charity Navigator's top 4-star rating two years in a row, as well as a perfect 100 percent score for governance and transparency for the last seven years.

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