

2023 CMTA-STAR

CELEBRATING OUR COLLECTIVE ACHIEVEMENTS Making Strides Together in CMT Research

Significant Research Investment Made in 2023: We are excited to share that in 2023, the CMTA has ongoing and committed investments of \$5,000,000 in research dedicated to finding effective treatments and ultimately a cure for Charcot-Marie-Tooth disease.

Support for Natural History Studies Drives Clinical Trial Readiness: In 2023, we approved funding for multiple new sponsored research projects, including a multi-year commitment to study natural history in CMT1A patients. This commitment for \$1.2 million will support measures such as biomarkers designed to help clinical trials get to faster outcomes. Our natural history studies in 1B, 1X, 2A, and 2F are ongoing, with over \$2 million in support, and help to understand the progress of CMT in these subtypes.

We are proud to announce that the first clinical trial using MRI as a primary outcome measure commenced recruitment in 2023, with funding from the CMTA.

Strong Advancements Made in Genetic Therapy: The CMTA is currently funding the development of genetic therapy across all major CMT subtypes! This includes projects supporting gene editing for demyelinating (Type 1) CMTs and a genetic therapy for 4A. We've initiated our first project to develop a genetic therapy for 2E, and we've also sponsored a nanoparticles project to help delivery of drugs all the way to the ends of the peripheral nerves.

Pre-Clinical Testing Partnerships Support Research of New Treatments: With our STAR Alliance Partners, we are investing over \$2 million in 2023 for pre-clinical testing. These efforts encompass 17 studies across 1A, 2A, and 2E. And we are very proud to report that a patent has been awarded for the CMTA-developed CMT2A rat model.

Industry Investment Continues to Grow with Our Support: Our ecosystem, research, and our toolbox developed over 15 years of STAR have created an environment where industry investment has reached new heights in 2023, and led to an increasing number of treatments heading to clinical trials. Notably, Novartis acquired DTx Pharma's CMT1A treatment and will move it forward into the clinic.

The CMTA's STAR Investment arm also made its first investment in Armatus Bio. This type of investment gives us an opportunity to support drug development for CMT directly in biotechnology companies.

Community Engagement Advances Drug Development: With over 6,400 community members having profiles in Patients as Partners in Research, we have supported recruitment for more than 23 studies, surveys, and focus groups to advance the development of CMT treatments.

On the Hunt for New Genes: Continued funding of the GENESIS project has supported the discovery of mutations in 131 genes causing 165 CMT subtypes. In 2023 alone, we have identified 5 new CMT-causative mutations.

These accomplishments are a testament to what we can achieve when we stand united against CMT. Your unwavering support, dedication, and passion have made all of this possible. Together, we are making a significant impact on the journey to conquer CMT.