CMT Research Foundation Provides Funding to Four Innovative Projects Through Emerging Researcher Award

Funding supports early-career scientists pursuing high-impact CMT drug development research

ATLANTA, GA, UNITED STATES, September 10, 2025 /EINPresswire.com/ -- The CMT Research Foundation has awarded funding to four new research projects through its Emerging Researcher Award program, which provides small, high-impact grants to promising scientists in academia and industry. Spanning multiple Charcot-Marie-Tooth disease subtypes, these awards enable early-career investigators to tackle key challenges in drug development and generate the preliminary data needed to advance future therapies.

Two types of awards are offered: Predoctoral Grants, open to current PhD and MD students, and Pilot Grants, open to postdoctoral fellows and early-career investigators.

“These four projects represent bold, transformative ideas that could pave the way for new therapies and drive innovation in CMT research,” said Laura M. MacNeill, CEO of the CMT Research Foundation. “By funding the next generation of researchers, we’re not only advancing our understanding of CMT but also investing in the future of treatments that could improve the lives of patients and families affected by this disease.”

Jonathan Alevy (Predoctoral Grant)

Alevy is investigating how mutations in the TRPV4 (transient receptor potential vanilloid 4) gene disrupt neurovascular endothelial cells and contribute to CMT2C. His work is connected to ongoing efforts to develop TRPV4-targeted therapies in collaboration with Actio Biosciences, highlighting its translational potential. Using single-cell RNA sequencing in mutant mouse models, his project aims to identify changes in gene expression in neurovascular endothelial cells that could serve as biomarkers to monitor treatment efficacy for the ActioBio compound and reveal additional therapeutic targets. By studying how mutant TRPV4 affects endothelial cell function, the research could guide future clinical strategies and improve patient monitoring.

Alevy is a PhD candidate at Johns Hopkins University in Dr. Charlotte Sumner’s lab, where his research focuses on understanding mechanisms of neurovascular dysfunction in neurodegenerative disease.

Daniel Bouch (Predoctoral Grant)

Bouch is engineering next-generation viral vectors, called adeno-associated viruses, to deliver gene therapies directly to Schwann cells. Schwann cells are critical for producing myelin and maintaining peripheral nerve function. His project, under the guidance of Dr. Afrooz Rashnonejad, aims to overcome the major challenge of crossing the blood-nerve barrier to efficiently reach Schwann cells. Using a “Key-Switch” system, his team will generate a library of AAVs displaying different surface “keys,” each with a “switch” that activates only when the virus successfully reaches Schwann cells. This approach allows the researchers to identify which “keys” most effectively target Schwann cells and understand the biological pathways — or “locks” — that enable delivery across the blood-nerve barrier. By optimizing these delivery vehicles, the project could improve gene therapy strategies for demyelinating forms of CMT and inform future approaches for targeted nerve repair.

Bouch is a PhD candidate at The Ohio State University in Dr. Afrooz Rashnonejad’s lab, where he focuses on engineering next-generation gene therapy vectors with enhanced tissue tropism, aimed at translating CMT therapies into clinical applications.

Mary Kate McCulloch (Predoctoral Grant)

McCulloch is developing patient-derived organoids, which are miniature lab-grown models of the peripheral nervous system. She will use this approach to better understand how genetic mutations causing CMT1B affect nerve cells and test a novel gene therapy designed to address the underlying genetic mutation. Her project, under the guidance of Dr. Afrooz Rashnonejad, aims to characterize patient-derived peripheral nerve organoids (PNOs) to elucidate molecular pathomechanisms caused by mutations in the MPZ gene, and to evaluate the efficacy of knockdown and replacement gene therapy in early- and late-onset CMT1B PNOs. By advancing their understanding of CMT1B and providing a platform for testing potential treatments, the development of PNO models could aid in treatment development for many CMT subtypes.

McCulloch is a PhD candidate at The Ohio State University in Dr. Afrooz Rashnonejad’s lab, which is pioneering the first gene therapy for CMT1B.

Sarah Raissi, PhD (Pilot Grant)

Dr. Raissi is exploring how CMT causes motor or sensory dysfunction through damage to peripheral neurons or myelin, the supportive coating that speeds nerve signaling. She is focusing on multiple forms of CMT in which myelin is damaged due to mutations in genes involved in the phosphoinositide signaling pathway, which is critical for proper myelin formation and maintenance. Dr. Raissi will test the hypothesis that loss of SMIT1 impairs phosphoinositide signaling in Schwann cells, leading to CMT-like myelin deficits, and that restoring this signaling can rescue these deficits. This project will investigate how the SMIT1 signaling pathway operates during normal formation and maintenance of myelin to better understand how these processes

may be compromised in CMT diseases.

Dr. Raissi received her PhD from Harvard Medical School and currently is a postdoctoral fellow in Dr. Jonah Chan's lab at UCSF where she is investigating molecular mechanisms of Schwann cell myelination, mastering both in vitro and in vivo genetic manipulation techniques, and generating novel mouse models to study inositol transporter function in the peripheral nervous system.

This inaugural round of Emerging Researcher Awards marks a significant step in expanding the pipeline of discovery for CMT. Each of these projects will provide milestone-driven updates, ensuring accountability and steady progress as the work advances toward potential treatments for patients and families living with CMT.

About the CMT Research Foundation: The CMT Research Foundation is a patient-led, non-profit organization dedicated exclusively to funding research that will lead to treatments and a cure for Charcot-Marie-Tooth disease. By focusing on high-impact, results-driven research, CMTRF partners with leading scientists, biotech companies and investors to bring promising therapies to clinical trials faster. For more information, visit www.cmtrf.org.

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