Leading Scientists and Advocates Unite for 'OHio, What a Night' to Accelerate Charcot-Marie-Tooth Disease Research

CMT Research Foundation Hosts Exclusive Event Spotlighting Groundbreaking Advances in CMT Treatment in Columbus

COLUMBUS, OH, UNITED STATES, March 27, 2025 /EINPresswire.com/ -- The CMT Research Foundation is bringing together world-renowned scientists, patient advocates and the Charcot-Marie-Tooth disease (CMT) community for OHio, What a Night, an inspiring evening dedicated to accelerating research and treatment development for CMT. The event takes place on Thursday, April 10, in Columbus, Ohio, spotlighting the region's pivotal role in cutting-edge CMT research.

A highlight of the evening is a keynote presentation by Dr. Jerry Mendell, a leading gene therapy pioneer and an advisor to the Jerry R. Mendell Center for Gene Therapy at the Abigail Wexner Research Institute at Nationwide Children's Hospital and Sarepta Therapeutics. Best known for his groundbreaking work in developing gene therapies for Duchenne muscular dystrophy and spinal muscular atrophy, Dr. Mendell will share insights into how gene therapy — driven by world-class researchers in Columbus — is shaping the future of CMT treatment.

"We are honored and excited to welcome Dr. Mendell to inspire greater momentum in innovation for CMT, which today has significant unmet needs," said Michael Triplett, Ph.D., event co-chair; co-founder and chair of Armatus Bio; and co-founder and board member of Neucore Bio. "His transformative work in gene therapy has already changed the lives of patients with devastating neuromuscular diseases, and we believe his expertise could be the key to unlocking long-awaited treatments — and ultimately, a cure — for CMT."

CMTRF is currently funding three gene therapy projects at Nationwide Children's Hospital:
· Development of a gene therapy for CMT1A, the most prevalent subtype of the disease, in collaboration with Armatus Bio.
· Development of a gene therapy for CMT1B, another subtype of the disease.
· Development of next-generation gene therapy delivery vehicles with improved activity in the cell types impacted in CMT — a breakthrough that could improve gene therapies across many CMT types.

"Powered by experts at Nationwide Children's Hospital and The Ohio State University, Columbus is at the forefront of cutting-edge CMT research," said Susan Ruediger, CMTRF co-founder and

chief mission officer. “The work being done here — fueled by CMTRF funding and led by experts like Dr. Mendell — is driving real progress toward life-changing breakthroughs for the CMT community.”

Charcot-Marie-Tooth disease is a progressive, degenerative disease affecting the peripheral nerves, impacting approximately 150,000 people in the U.S. and nearly 3 million worldwide. It is the most common inherited neuropathy but can also arise from spontaneous genetic mutations. There are currently no FDA-approved treatments or cures for CMT.

Proceeds from OHio, What a Night will directly fund research aimed at developing life-changing treatments and, ultimately, a cure for CMT.

For more information or to purchase tickets, visit cmtrf.org/events.

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.

About Nationwide Children’s Hospital
Nationwide Children’s Hospital, based in Columbus, Ohio, is one of the largest and most comprehensive pediatric hospitals and research institutes in the country. Home to the Abigail Wexner Research Institute, Nationwide Children’s is at the forefront of groundbreaking gene therapy and neuromuscular disease research, advancing innovative treatments for conditions like Charcot-Marie-Tooth disease, Duchenne muscular dystrophy and spinal muscular atrophy. Learn more at www.nationwidechildrens.org.

About The Ohio State University
The Ohio State University, founded in 1870, is one of the nation’s leading public research institutions, known for its excellence in academics, innovation and community impact. With its main campus in Columbus and regional campuses across Ohio, the university serves more than 60,000 students across a wide range of undergraduate, graduate and professional programs. To learn more, visit www.osu.edu.

About Armatus Bio
Armatus Bio is a late-preclinical stage, privately held biotech innovator developing vectorized RNAi medicines in neuromuscular disorders. Its lead candidate, TVR110, is in development for Charcot-Marie-Tooth disease type 1A (CMT1A), a progressively debilitating disorder with no approved treatments today. The engineered miRNA product is designed to reduce PMP22 overexpression to healthy levels and is on track to begin clinical trials by 2026. Armatus is also advancing a second asset for Facioscapulohumeral Muscular Dystrophy (FSHD). For more

information, visit www.ArmatusBio.com.

About Sarepta Therapeutics
Sarepta is on an urgent mission: engineer precision genetic medicine for rare diseases that devastate lives and cut futures short. We hold leadership positions in Duchenne muscular dystrophy (Duchenne) and limb-girdle muscular dystrophies (LGMDs) and are building a robust portfolio of programs across muscle, central nervous system, and cardiac diseases. For more information, please visit www.sarepta.com.

Kayleena Speakman
CMT Research Foundation
+1 404-806-7180
kayleena.speakman@cmtrf.org
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