

CMTA Announces Participation in Seed Extension Round for Armatus Bio for Novel CMT1A Gene Therapy

First Investment from CMTA's New Venture Philanthropy Arm

GLENOLDEN, PA, USA, November 14, 2023 /EINPresswire.com/ -- The Charcot-Marie-Tooth Association (CMTA) today announced that it participated in the Seed Series Extension syndicate for Armatus Bio, Inc. with an undisclosed investment.



Armatus is an emerging biotechnology company based in Columbus, Ohio, that is advancing a unique gene therapy clinical candidate to target CMT type 1A, the most common form of the disease.

Charcot-Marie-Tooth (CMT) disease is an inherited peripheral neuropathy that principally affects the long nerves of the hands and feet. CMT has an incidence of one in 2,500 people, or 3 million people, worldwide. As nerves are damaged with disease progression, surrounding muscles atrophy, resulting in profound weakness and loss of balance, often requiring surgery, orthotics and eventually additional aids such as wheelchairs to maintain mobility. Despite the high prevalence of CMT and its often-predictable pattern of inheritance in affected families, the disease is frequently misdiagnosed or not diagnosed promptly. Currently, there are no FDA-approved therapies for any type of CMT.

CMT1A is caused by duplication of the PMP22 gene. PMP22 is a protein produced by Schwann cells and is a component of the myelin sheath surrounding nerves. Overexpression of PMP22 among individuals with CMT1A results in the destabilization and loss of the myelin sheath, leading to increasing nerve loss over time and disease progression. The Armatus treatment—ARM-101—is an adeno-associated virus (AAV) based gene therapy that encodes a small interfering RNA (siRNA) that works by lowering the production of PMP22 protein in Schwann cells by the targeted degradation of PMP22 mRNA. Armatus has already demonstrated the efficacy of the approach in CMT1A mouse models and is currently conducting studies that will enable the company to submit an IND in 2025 to the FDA, which if cleared will result in a Phase 1 clinical trial to evaluate the safety, tolerability and possible clinical benefit of ARM-101 in

patients with CMT1A. In addition ARM-1, Armatus is collaborating with renowned gene therapy experts in Ohio to build a pipeline of novel therapeutic candidates designed to overcome the limitations of today's approaches and propel the next generation of genetic medicines.

Armatus CEO Rachel Salzman, DVM, commented, "Armatus is extremely grateful to the CMTA for its support, and we look forward to their continued advice and counsel as we advance ARM-101." Dr. Salzman went on to say, "Our ARM-101 development studies are particularly important for the CMT community because the data generated are designed to answer critical questions about how we can effectively deliver genetic medicines to the right parts of the body to meaningfully address this disease." She added, "As we accelerate development of ARM-101 and gain further evidence that our vectorized RNAi can normalize PMP22 in Schwann cells as a means of enabling human trials, we believe this therapy has the potential to provide benefit to the thousands of people living with CMT1A in the U.S. and around the world."

Thomas Dubensky, Ph.D., a CMTA board member commented "The CMTA is very excited to facilitate the advancement of novel therapies to be ultimately evaluated in patients living with all types of CMT. Armatus is the CMTA's first investment from its new venture philanthropy arm, which will make selected early-stage investments in Biotech companies developing unique and promising therapies against all types of CMT." Dr. Dubensky went on to say that "the venture philanthropy arm will work in tandem with STAR (Strategy to Accelerate Research), the arm of CMTA which is the largest private funder of CMT research in the world."

About the CMTA:

The vision of the CMTA is a world without CMT. The CMTA is the leading patient advocacy organization driving CMT research. Its mission is to develop treatments and a cure for the 3 million people worldwide who live with the disease, while improving the quality of life for patients and their families. The CMTA has invested \$23.5 million in the search for a cure—the largest philanthropic funder in the world—and its Strategy to Accelerate Research (STAR) brings world-renowned CMT researchers together with pharmaceutical and biotechnology partners and patients to accelerate scientific breakthroughs. The CMTA also offers community services, including 70 local branches, educational materials and patient conferences. Of note, the CMTA has a perfect 100 4-star ranking granted by Charity Navigator.

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