

CMTA AND APPLIED THERAPEUTICS ANNOUNCE COLLABORATION FOR CMT-SORD

GLENOLDEN, PA, USA, October 26, 2021 /EINPresswire.com/ -- The Charcot-Marie-Tooth Association (CMTA), the largest philanthropic funder of CMT research worldwide, and Applied Therapeutics, a clinical-stage biopharmaceutical company developing a pipeline of novel drug candidates against validated molecular targets in indications of high unmet medical need, today announced a collaboration to investigate a potential therapy for a newly discovered type of the disease caused by a deficiency of the SORD gene.

The primary goal of the collaboration is to help identify patients who may be eligible to participate in upcoming clinical trials and provide insight into clinical trial planning through the CMTA's Patients as Partners in Research initiative. Through this collaboration, patients will be able to get free SORD testing by having a nurse come to their home or through their doctor's office, and the patient perspective will be shared to help shape trials to come.

A team led by Dr. Stephan Züchner at the University of Miami discovered SORD. Züchner's team, including Drs. Andrea Cortese, Grace Zhai, Adriana Rebelo and many others, found that mutations in the SORD gene cause an axonal form of CMT that is recessive. The newly discovered type is caused by a mutated SORD (sorbitol dehydrogenase) gene that raises sorbitol levels so high they cause nerve damage. Researchers found that treating fruit flies with a type of drug called an aldose reductase inhibitor, reduced their high levels of sorbitol to near normal. It is estimated that at least 3,000 –4,000 people in the United States and 4,000 patients in Europe, have this type of CMT, making it the most common recessive form of the disease.

AT-007 is an oral aldose reductase inhibitor in development for SORD. Because the drug is still in clinical trials, it's called an "investigational drug." AT-007 blocks the enzyme that precedes sorbitol dehydrogenase to prevent sorbitol from being formed in the body. In a recent pilot study in 8 SORD patients, AT-007 reduced sorbitol levels by 66% in the blood, with a range of individual patient reductions of 54%-75%. AT-007 has also been studied in healthy volunteers as well as adults and children with another rare disease called Galactosemia. Applied Therapeutics is planning to initiate a larger registrational study in SORD patients with sites in the US and Europe towards the end of this year. The hope is that the registrational trial will support approval of the first drug to treat SORD.

The collaboration with Applied Therapeutics is part of the CMTA's Strategy to Accelerate Research (STAR), which connects leading CMT clinicians and researchers with pharmaceutical partners

committed to developing treatments and a cure for CMT. Strategic alliances with pharmaceutical partners like Applied Therapeutics support drug development efforts to deliver therapies to CMT patients.

ABOUT CMT

Charcot-Marie-Tooth disease or CMT is named after the three physicians who first described it: Drs. Jean-Martin Charcot, Pierre Marie, and Howard Henry Tooth. CMT is a group of like diseases caused by inherited genetic mutations that damage the peripheral nerves outside of the brain and spinal cord. Scientists have identified over 100 different gene mutations causing CMT. Most people (90%) have one of four types of CMT: CMT 1A (PMP 22); CMT 1B (MPZ); CMT 2A (MFN2) and CMT 1X (GJB1). It is estimated that CMT affects 3+ million people worldwide, regardless of gender, race, or ethnicity. A tremendous amount of awareness about CMT was created recently when country music legend Alan Jackson announced he has the disease. The CMTA firmly believes that awareness like this will lead to more progress in the pursuit to develop treatments for the disease. <https://www.cmtausa.org/understanding-cmt/what-is-cmt/>

ABOUT THE CMTA

The CMTA is the largest philanthropic funder of CMT research worldwide. The CMTA's Strategy to Accelerate Research (STAR) brings the best CMT researchers, clinicians, and experts in therapy development together with pharmaceutical and biotechnology companies and patients to expedite the development of treatments for CMT. The CMTA is also actively working to help improve the quality of life for all families living with CMT by offering educational programs and materials, hosting patient and professional conferences, providing support to families through its nationwide branch system through North America and more. More information can be found at www.cmtausa.org.

ABOUT APPLIED THERAPEUTICS

Applied Therapeutics is a clinical-stage biopharmaceutical company developing a pipeline of novel drug candidates against validated molecular targets in indications of high unmet medical need. The Company's lead drug candidate, AT-007, is a novel central nervous system penetrant Aldose Reductase Inhibitor (ARI) for the treatment of CNS rare metabolic diseases, including Galactosemia, SORD Deficiency and PMM2-CDG. The Company is also developing AT-001, a novel potent ARI, for the treatment of Diabetic Cardiomyopathy, or DbCM, a fatal fibrosis of the heart. The preclinical pipeline also includes AT-003, an ARI designed to cross through the back of the eye when dosed orally, for the treatment of diabetic retinopathy, as well as novel dual PI3k inhibitors in preclinical development for orphan oncology indications.

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