

CMTA AND TOOLGEN ANNOUNCE COLLABORATION TO DEVELOP POTENTIAL TREATMENT FOR CMT1A USING GENE THERAPY

GLENOLDEN, PA, USA, October 7, 2021

/EINPresswire.com/ -- The

Charcot-Marie-Tooth Association (CMTA), the largest philanthropic funder of CMT research worldwide, and ToolGen, a biotechnology company focusing on development of the genome editing platform technology and its applications in

human therapeutics and agriculture, today announced a collaboration to investigate a potential therapy for CMT type 1A (CMT1A), the most common subtype of the disease, which affects approximately 1.5 million people, using CRISPR gene editing technology.



The primary goal of the collaboration is to facilitate the development of a novel gene-editing therapy for CMT1A that seeks to suppress overactivity of the causative disease gene, PMP22.

The CMTA's Strategy to Accelerate Research (STAR) connects leading CMT clinicians and researchers with pharmaceutical partners committed to developing treatments and a cure for CMT. Strategic alliances with pharmaceutical partners like ToolGen support drug development efforts to deliver therapies to CMT patients.

"We are excited to establish this partnership with ToolGen as they work to advance gene editing technology to treat CMT1A," said CMTA's CEO Amy Gray. "Developing strategic research partnerships with companies like ToolGen is a central part of our strategy to accelerate treatments for the CMT community. Since launching STAR 12 years ago, the CMTA has been able to establish research partnerships with almost 40 pharmaceutical and biotech companies, and leading research labs around the world."

The testing alliance with ToolGen involves access to the STAR Scientific and Clinical advisors of the CMTA, animal testing of the approach in the CMTA preclinical testing alliance network, and assistance with the planning of clinical trials in the USA. The CMTA's preclinical testing alliance:

www.cmtausa.org/our-research/for-researchers/cmta-preclinical-testing-network/ makes this possible.

“We are delighted to have this collaboration to strengthen our drug development program for CMT1A where no approved therapy is available,” said ToolGen’s Director of R&D Therapeutics Jae young Lee. “This alliance with the Charcot-Marie-Tooth Association will enable us thorough testing of our drug candidate in suitable CMT1A models and lead us closer to initiating clinical trials.”

Interview with ToolGen related to this can be found: https://www.youtube.com/watch?v=-jyb7Dxw_al&feature=youtu.be

ABOUT CMT

CMT is the acronym for Charcot-Marie-Tooth disease, named after the three physicians who first described it: Drs. Jean-Martin Charcot and 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 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 TOOLGEN

ToolGen, Inc. is a publicly traded biotechnology company focused on the development and application of genome editing technologies. It creates and holds intellectual property rights for essential tools and technologies for editing the genetic information in microbial, plant, animal, and human cells. ToolGen’s mission is to translate the potential of our innovative platform technology into transformative products for biomedicine and agriculture. For more information, please visit eng.toolgen.com

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