Shift Pharmaceuticals, Charcot-Marie-Tooth Association Partner on ASO Treatment for CMT1A

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Shift Pharmaceuticals, a privately held company that is developing antisense oligonucleotides (ASOs) to treat a variety of genetic disorders, and the Charcot-Marie-Tooth Association (CMTA) today announced a collaboration that will use ASOs to treat CMT1A. Type 1A is caused by a duplication in the PMP22 gene. ASOs are drugs that can alter RNA and reduce, restore or modify protein expression.

The collaboration takes advantage of the CMTA's extensive suite of expert preclinical testing capabilities for companies like Shift Pharmaceuticals that want to evaluate the therapeutic potential of a drug candidate. The findings from the project will be critical in advancing research that will lead to clinical trials for CMT1A patients.

CMT is a degenerative neuromuscular disease that entered the national spotlight last year when country music legend Alan Jackson announced he has the disease. Symptoms include muscle weakness, decreased muscle size, foot drop, foot bone abnormalities, fatigue, balance problems, neuropathic and/or musculoskeletal pain, loss of feeling in the hands and feet and loss of coordination in the limbs. There are no Food and Drug Administration-approved treatments to stop or reverse the loss of nerve function in CMT.

"Our entire team is excited to be working with the CMTA on the development of our lead candidate for CMT1A, SHC1A-012. Their expertise in pre-clinical development and funding natural history studies in this disease will greatly assist with the acceleration of our program to the preparation of the final Investigational New Drug application and filing with the FDA," said Steve O'Connor, CEO of Shift Pharmaceuticals. "Additionally, we feel strongly that their efforts in patient engagement will provide invaluable guidance in designing clinical outcomes to maximize the benefit for the entire CMT1A patient community."

CMTA CEO Amy Gray said, "We are thrilled to enter into this collaboration with Shift Pharmaceuticals to broaden the work taking place to develop treatments for the CMT community."

ABOUT CMT
CMT is a group of 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 percent) 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 more than 3 million people worldwide, regardless of gender, race, or ethnicity.
https://www.cmtausa.org/understanding-cmt/what-is-cmt/

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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. Since 2008, the CMTA has invested more than $18.5 million in STAR, with plans to invest another $10 million in the next few years. 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.

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