



Clinical Trial Explores New CMT1A Drug Therapy

A first-of-its-kind treatment may be on the horizon for people living with Charcot-Marie-Tooth (CMT) disease.

CMT is an umbrella under which many subtypes of the disease are classified. CMT1A is characterized by issues with myelin, the protective substance that insulates nerves. In patients with CMT1A, decreased myelin slows impulse transmission, which can lead to muscle weakness. CMT1A is the most common subtype of CMT and [affects about half](#) of people with CMT.

Currently, CMT1A patients might take medication to manage nerve pain, use orthopedic devices to maintain mobility, and/or undergo physical therapy to strengthen muscles, but there is no available treatment to address the underlying cause of the disease.

Researchers are now exploring a combination therapy that aims to improve symptoms of CMT1A by tackling the cause at the cellular level. In patients with CMT1A, the overexpression of PMP22 protein causes abnormal myelin production. A [phase 3 clinical trial](#) sponsored by Tasly GeneNet Pharmaceuticals Co., Ltd, is testing the efficacy and safety of [PXT3003](#), a novel combination of baclofen, naltrexone, and sorbitol, to reduce the overexpression of PMP22 protein and improve neural signals in peripheral nerves.

“This is exciting because this could be the first specific treatment option for CMT1A,” says Jaclyn Omura, MD, a pediatric rehabilitation medicine physician and Co-Director of the MDA Care Center at Seattle Children’s Hospital. “We can offer medications for nerve pain, but they’re not specifically for CMT. This trial is targeting symptoms of CMT1A, which can limit things like the distance a person can walk or daily living activities.”

The trial is actively recruiting through the end of 2023. Interested candidates must be between 16 and 65 years old and have a confirmed case of CMT1A, among other criteria. Over the course of the randomized, double-blind, placebo-controlled study, researchers will be looking for improvements in participants’ Overall Neuropathy Limitation Scale (ONLS) score.

