Research News and Updates

HELIOS WOLFRAM SYNDROME CLINICAL TRIAL UPDATE-AMYLX PHARMACEUTICALS

The Amylyx HELIOS trial is a 12-participant, single-site, open-label, Phase 2 clinical trial designed to evaluate the safety and tolerability of AMX0035, an investigational medicine, and various measures of endocrinologic, neurologic, and ophthalmologic function in adult participants living with Wolfram syndrome. In October 2024,
Amylyx announced positive topline results from HELIOS (NCT05676034). The primary endpoint of HELIOS is measuring C-peptide response, which is a measure of the ability of the pancreas to produce insulin. After 24 weeks of treatment with AMX0035, improvement in C-peptide response was observed, which means that pancreatic function improved. AMX0035 was generally well-tolerated. All adverse events (AEs) were mild or moderate, and no serious

AEs were related to AMX0035 treatment.

HELIOS is no longer enrolling participants but remains ongoing for those already enrolled. Limitations of HELIOS include that it is a small study and there is no placebo group to compare the AMX0035 group to. Thus, AMX0035 will need to be studied further in Wolfram syndrome in a Phase 3 program. Amylyx continues to engage with FDA and other stakeholders to inform a Phase 3 program and expects to provide an update in 2025.

Living with Wolfram Syndrome: Sharing Raquel's Story

Raquel and her mother, Stephane Gebel, recently had the opportunity to share Raquel's story about living with Wolfram syndrome, how it's impacted Raquel's childhood and college experience, and what we'd like those who are researching Wolfram syndrome to know in order to make a meaningful difference for our community. Check out the resulting video here to hear their perspectives: https://www.amylyx.com/wolfram-syndrome

San Raffaele Scientific Institute (Italy) - This project will evaluate the use of gene therapy as a treatment for WS, and evaluate the role that inflammation plays in WS. They plan to deliver functioning, wild-type WFS1 protein to WS mutant precursor white blood cells. This delivery will occur in a mouse model with humanized beta (insulin producing) cells. In doing so they hope to decrease inflammation in WS, and also further refine a method of delivering wild-type WFS1 to WS mutant cells through gene therapy.

University of Tartu (Estonia) - Investigations have been completed determining the effects of liraglutide, Gaba, and taurine in WS cells and mice. TSF plans to continue partnering with Estonian researchers to expand the search for repurposed therapeutics and small molecules supporting cell function in WS.

Washington University in St. Louis
School of Medicine

Washington University School of Medicine (USA) - MANF preclinical data collection is ongoing by Dr. Fumihiko Urano, who is using MANF as a peptide to support retinal cell regeneration. TSF is currently focused on facilitating the development of MANF as a WS therapeutic. We aim to help get MANF from the lab to clinical trial for WS. We are also evaluating the safety and effectiveness of combination therapy with Amylyx, alongside Liraglutide or Mounjaro.