



Amylyx Pharmaceuticals to Host Virtual Webcast to Discuss Interim Data from Phase 2 HELIOS Study of AMX0035 in Wolfram Syndrome on April 10, 2024

April 8, 2024 at 9:00 AM EDT

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Apr. 8, 2024-- Amylyx Pharmaceuticals, Inc. (Nasdaq: AMLX) ("Amylyx" or the "Company") today announced the Company will host a virtual webcast with management and Fumihiko Urano, MD, PhD, Principal Investigator of the HELIOS clinical trial and the Samuel E. Schechter Professor of Medicine in the Division of Endocrinology, Metabolism & Lipid Research at Washington University School of Medicine in St. Louis to discuss interim data from HELIOS, a Phase 2 trial of AMX0035 for the treatment of Wolfram syndrome on April 10, 2024 at 1:30pm ET.

A live webcast of the presentation can be accessed under "Events and Presentations" in the Investor section of the Company's website, <https://investors.amylyx.com/news-events/events>, and will be available for replay for 90 days following the event.

Dr. Fumihiko Urano is a physician and medical researcher specializing in Wolfram syndrome. Dr. Urano serves as a Professor of Medicine and of Pathology & Immunology and is the Samuel E. Schechter Professor of Medicine at Washington University School of Medicine in St. Louis. Dr. Urano is a driving force in the study of Wolfram syndrome and related disorders, including WFS1-related disorders/Wolfram-like disorders. As director of the Wolfram Syndrome Clinic and the Wolfram Syndrome International Registry & Clinical Study at Washington University, Dr. Urano treats patients with Wolfram syndrome and related disorders, and leads basic science, clinical, translational, and interventional studies of Wolfram syndrome and related disorders.

About the HELIOS Trial

The HELIOS trial ([NCT05676034](https://clinicaltrials.gov/ct2/show/study/NCT05676034)) is a 12-participant, open-label, proof of biology, Phase 2 trial designed to study the effect of AMX0035 on safety and tolerability, and various measures of endocrinological, neurological, and ophthalmologic function in adult participants living with Wolfram syndrome.

About Wolfram Syndrome

Wolfram syndrome is an autosomal recessive neurodegenerative disease characterized by childhood-onset diabetes, optic nerve atrophy, and neurodegeneration. Common manifestations of Wolfram syndrome include diabetes mellitus, optic nerve atrophy, central diabetes insipidus, sensorineural deafness, neurogenic bladder, and progressive neurologic difficulties. Genetic and experimental evidence suggests that endoplasmic reticulum (ER) dysfunction is a critical pathogenic component of Wolfram syndrome. The prognosis of Wolfram syndrome is poor, and many people with the disease die prematurely with severe neurological disabilities.

About AMX0035

AMX0035 is an oral, fixed-dose combination of sodium phenylbutyrate (PB) and taurursodiol (TURSO; also known as ursodoxicoltaurine outside of the U.S.). AMX0035 was designed to slow or mitigate neurodegeneration by targeting endoplasmic reticulum (ER) stress and mitochondrial dysfunction, two connected central pathways that lead to cell death and neurodegeneration. We believe that our proprietary combination of PB and TURSO and their complementary mechanisms of action will allow us to synergistically target abnormal cell death to better prevent neurodegeneration than treatment targeted at either mechanism of action alone. AMX0035 is being studied as a potential treatment for Wolfram syndrome and progressive supranuclear palsy, two neurodegenerative diseases.

About Amylyx Pharmaceuticals

Amylyx Pharmaceuticals, Inc. is committed to supporting and creating more moments for the neurodegenerative disease community through the discovery and development of innovative new treatments. Amylyx is headquartered in Cambridge, Massachusetts. For more information, visit amylyx.com and follow us on [LinkedIn](#) and [X](#), (formerly Twitter). For investors, please visit investors.amylyx.com.

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