



Amylyx Pharmaceuticals to Host Virtual Webcast to Discuss Topline Results from Phase 2 HELIOS Study of AMX0035 in Wolfram Syndrome on October 17, 2024

October 15, 2024 at 9:00 AM EDT

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Oct. 15, 2024-- Amylyx Pharmaceuticals, Inc. (Nasdaq: AMLX) ("Amylyx" or the "Company") today announced the Company will host a virtual webcast on October 17, 2024 at 1:30 p.m. ET 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 (WashU Medicine), to discuss topline results from HELIOS, a Phase 2 trial of AMX0035 (sodium phenylbutyrate [PB] and taurursodiol [TURSO, also known as ursodoxicoltaurine]) for the investigational treatment of Wolfram syndrome. The live webcast follows the presentation of these data at the International Society for Pediatric and Adolescent Diabetes 50th Annual Congress in Lisbon, Portugal.

The live webcast 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 leading medical researcher at WashU Medicine in St. Louis specializing 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 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 is committed to the discovery and development of new treatment options for communities with high unmet needs, including people living with serious and fatal diseases. The Company has preclinical or clinical development programs underway in neurodegenerative, neuroendocrine, and endocrine diseases. Since its founding, Amylyx has been guided by science to address unanswered questions, keeping communities at the heart and center of all decisions. Amylyx is headquartered in Cambridge, Massachusetts. For more information, visit amylyx.com and follow us on [LinkedIn](#) and [X](#). For investors, please visit investors.amylyx.com.

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