



Amylyx Pharmaceuticals Receives FDA Orphan Drug Designation for AMX0035 for the Treatment of Amyotrophic Lateral Sclerosis

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NEW YORK AND CHICAGO, Oct. 24, 2017 /PRNewswire-USNewswire/ — The Alzheimer's Association and Alzheimer's Drug Discovery Foundation (ADDF) are collaborating to jointly fund a new combination therapy clinical trial for Alzheimer's disease to be conducted by Amylyx Pharmaceuticals. The \$1.85 million grant is the first award under an initiative created by the Alzheimer's Association and the ADDF to fund combination therapies.

The grant will support a phase 2 clinical trial of AMX0035, a combination of sodium phenylbutyrate (PB) and tauroursodeoxycholic-acid (TUDCA). The trial, expected to begin in the first half of 2018, will include approximately 50 people with mild cognitive impairment or mild-to-moderate Alzheimer's disease and test the drug's effectiveness at slowing or stopping brain cell death.

"Combination therapies hold great potential to slow the progression of Alzheimer's," said Howard Fillit, MD, the ADDF's founding executive director and chief science officer. "The innovation of Amylyx's combination therapy is that it targets multiple causes of brain cell loss, and the two drugs given in tandem create additional protective effects. The ADDF was an early supporter of innovative targets for Alzheimer's, and we believe combination therapies are a critical next step in finding effective treatments for the disease."

Alzheimer's is a complex disease with multiple, interrelated causes. A growing number of experts believe combination therapy—with two or more drugs, or drugs and lifestyle interventions—will be required to effectively treat it.

"We have witnessed the success of combination therapy in HIV/AIDS, cancer, and heart disease. There is strong reason to believe that to successfully address Alzheimer's, and its extraordinary complexity, we need to attack the disease on multiple fronts," said Maria Carrillo, PhD, Alzheimer's Association chief science officer. "Meetings convened in 2015 by the Alzheimer's Association ([proceedings](#) published in 2016) and the ADDF led the two organizations to recognize the potentially important role of combination therapies in Alzheimer's and paved the way for this exciting partnership and initial clinical trial funding."

New approaches to Alzheimer's are urgently needed, as deaths from the disease continue to rise precipitously as more and more Baby Boomers reach the age of greatest risk.

The grant was made through the Alzheimer's Combination Therapy Opportunities (ACTO) program, a joint research funding initiative created by the Alzheimer's Association and the ADDF to support clinical trials combining multiple treatment approaches. The ACTO program specifically called for study proposals using repurposed drugs that have been determined safe for use in treating other conditions. Repurposing may speed the drug development process because researchers can often begin with phase 2 trials including outcome measures of effectiveness, rather than phase 1 safety tests.

The drug to be tested, AMX0035, is a proprietary oral formulation of two existing therapeutics. PB is an FDA-approved therapy—currently prescribed for urea-cycle disorders—that activates genes responsible for protecting brain cells from toxic unfolded proteins. TUDCA is an acid produced in small amounts by the body that targets cellular energy loss. In preclinical studies conducted by the company and with academic collaborators, combination dosing of PB and TUDCA protected brain cells from inflammation and oxidation. Amylyx received FDA clearance for AMX0035's Investigational New Drug application in April 2017 and has an ongoing multicenter clinical study of the compound in people with amyotrophic lateral sclerosis (ALS).

"We are very grateful for the support from the Alzheimer's Association and the Alzheimer's Drug Discovery Foundation, and we're honored to partner with them in this new combination therapy initiative," said Kent Leslie, Amylyx chief scientific officer. "Through a combination approach targeting two different and independent pathways, AMX0035 is designed to benefit both neurodegeneration and neuroinflammation, key drivers of Alzheimer's and ALS. The biomarker-focused trial design will assist in translating the promising preclinical effects observed in models of Alzheimer's to an improved understanding of the potential of AMX0035 to help individuals living with this disease."

Alzheimer's Drug Discovery Foundation

Founded in 1998 by Leonard A. Lauder and Ronald S. Lauder, the Alzheimer's Drug Discovery Foundation is the only philanthropy solely focused on accelerating the development of drugs to prevent and treat Alzheimer's disease. Its venture philanthropy approach and scientific expertise allows the ADDF to support the most promising ideas around the world. And 100% of every donation funds drug research programs. To learn more, visit <http://www.alzdiscovery.org>.

Alzheimer's Association

The Alzheimer's Association is the leading voluntary health organization in Alzheimer's care, support and research. It is the largest nonprofit funder of Alzheimer's research. The Association's mission is to eliminate Alzheimer's disease through the advancement of research; to provide and enhance care and support for all affected; and to reduce the risk of dementia through the promotion of brain health. Its vision is a world without Alzheimer's. Visit alz.org or call 800.272.3900.

Amylyx Pharmaceuticals

Amylyx Pharmaceuticals, Inc. is a clinical-stage pharmaceutical company currently developing a novel therapeutic for Amyotrophic Lateral Sclerosis (ALS) and Alzheimer's disease. The company's therapeutic, AMX0035, targets the neuroinflammation and nerve cell death that characterize these diseases. AMX0035 is a proprietary combination of existing compounds that have worked synergistically to prevent cell death and neurotoxic inflammation in multiple preclinical models. AMX0035 entered a phase 2 clinical trial (CENTAUR) in ALS patients in June 2017. Learn more

at www.amylx.com.

SOURCE Alzheimer's Drug Discovery Foundation