



## Amylyx Pharmaceuticals Announces Last Patient Completes Final Study Visit in PEGASUS Trial of AMX0035 in Alzheimer's Disease

November 9, 2020

- *Topline Results Expected in the First Half of 2021*

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- Amylyx Pharmaceuticals, Inc., a pharmaceutical company focused on developing new treatments for amyotrophic lateral sclerosis (ALS), Alzheimer's disease and other neurodegenerative diseases, today announced that the last participant has completed the planned 24 weeks in the Phase II PEGASUS trial assessing the safety and biological activity of AMX0035 administration in people with Alzheimer's disease.

"Alzheimer's is a complex, progressive brain disease defined by amyloid and tau pathologies, but we now realize that there are multiple pathways driving the disease," said Steven E. Arnold, M.D., Professor and Translational Neurology Head of the Interdisciplinary Brain Center at Massachusetts General Hospital and Harvard Medical School and the PEGASUS trial principal investigator. "The PEGASUS trial design and broad inclusion criteria will allow us to assess whether AMX0035 can prevent neurodegeneration in people with Alzheimer's via MR imaging and fluid biomarkers. We are immensely thankful to the trial participants and their families for their time and commitment to this critical research effort."

In June 2020, Amylyx announced the PEGASUS trial completed enrollment and dosed 96 participants. Following brief disruptions due to the coronavirus pandemic, including trial site closures, the trial team worked to prioritize patient safety and mitigate delays by capping trial enrollment four patients shy of the planned 100. Amylyx worked with site investigators and clinic teams to facilitate remote visits and expand visit windows when possible. Topline results are targeted for the first half of 2021.

The PEGASUS trial will provide data on the safety and tolerability profile of AMX0035 in people with Alzheimer's disease, its biological activity on biomarkers related to disease processes and preliminary information on cognitive and functional effects of AMX0035.

Alzheimer's disease affects memory, thinking and behavior. Symptoms eventually become severe enough to interfere with daily tasks. Alzheimer's is a slow but relentlessly progressive disease. On average, a person with Alzheimer's lives four to eight years after diagnosis, but can live up to 20 years, depending on other factors. Alzheimer's disease is the sixth leading cause of death in the United States.

"Worldwide, 50 million people are awaiting new treatments for Alzheimer's disease, one of the most critically unmet healthcare needs today," said Dr. Rudolph Tanzi, Ph.D., Kennedy Professor of Neurology, Harvard Medical School, Vice-Chair of Neurology, Massachusetts General Hospital, Founding Chair of the Amylyx Scientific Advisory Board. "The PEGASUS trial will assess the impact of investigational product AMX0035 on biomarkers in people with Alzheimer's and hopefully lead to a future treatment. We are greatly looking forward to the data in the first half of 2021."

PEGASUS was supported by the Alzheimer's Combination Therapy Opportunities (ACTO) program, a joint research funding initiative supported by the Alzheimer's Association and the Alzheimer's Drug Discovery Foundation, and Cure's Alzheimer's Fund.

"We feel tremendous responsibility to all those living with Alzheimer's disease, their families and loved ones," said Kent Leslie, Chief Scientific Officer at Amylyx. "Amylyx is committed to rigorous scientific research to improve people's health, and we thank all patients, investigators, site teams, the Alzheimer's Association, the Alzheimer's Drug Discovery Foundation and Cure Alzheimer's Fund for their contributions to the PEGASUS study milestone. We look forward to sharing the topline results of the trial in the first half of 2021."

### **About PEGASUS**

PEGASUS ([NCT035333257](https://clinicaltrials.gov/ct2/show/study/NCT035333257)) is a 3:2 randomized, double-blind, multi-center, placebo-controlled study evaluating the safety, tolerability and activity of AMX0035 in patients with late mild cognitive impairment or early dementia due to Alzheimer's disease over 24 weeks. PEGASUS is designed to yield deep phenotyping insight for the purposes of demonstrating the effects of AMX0035 on mechanistic targets of engagement and disease biology. The study will evaluate diverse disease-relevant markers and produce an informative dataset that will allow for evaluation and correlation of imaging-based markers, neurobiological changes, functional measures, and cognitive outcomes.

### **About AMX0035**

AMX0035 is an investigational product designed to reduce neuronal death and dysfunction. AMX0035 targets endoplasmic reticulum and mitochondrial dependent neuronal degeneration pathways in amyotrophic lateral sclerosis (ALS), Alzheimer's disease and other neurodegenerative diseases.

### **About Amylyx Pharmaceuticals**

Amylyx Pharmaceuticals, Inc. is a pharmaceutical company working on developing a novel therapeutic for amyotrophic lateral sclerosis (ALS), Alzheimer's disease and other neurodegenerative diseases. For more information, visit [www.amylyx.com](http://www.amylyx.com) and follow us on [LinkedIn](#) and [Twitter](#).

### **Alzheimer's Association**

The Alzheimer's Association leads the way to end Alzheimer's and all other dementia — by accelerating global research, driving risk reduction and early detection, and maximizing quality care and support. Our vision is a world without Alzheimer's and all other dementia.™ For more information, visit [www.alz.org](http://www.alz.org) or call the 24/7 Helpline at 800.272.3900.

### **Alzheimer's Drug Discovery Foundation**

Founded in 1998 by Leonard A. and Ronald S. Lauder, the Alzheimer's Drug Discovery Foundation (ADDF) is dedicated to rapidly accelerating the discovery of drugs to prevent, treat and cure Alzheimer's disease. The ADDF is the only public charity solely focused on funding the development of

drugs for Alzheimer's, employing a venture philanthropy model to support research in academia and the biotech industry. Through the generosity of its donors, the ADDF has awarded more than \$150 million to fund over 626 Alzheimer's drug discovery programs and clinical trials in 19 countries. To learn more, please visit: <http://www.alzdiscovery.org/>.

**Cure Alzheimer's Fund**

Cure Alzheimer's Fund is a non-profit dedicated to funding the most promising research to prevent, slow or reverse Alzheimer's disease. Since its founding in 2004, Cure Alzheimer's Fund has contributed over \$110 million to research, and its funded initiatives have been responsible for several key breakthroughs – including the groundbreaking Alzheimer's in a Dish. Cure Alzheimer's Fund has received a 4 star rating for 9 consecutive years regarding its overall financial health from Charity Navigator. With 100 percent of donations going directly to research, Cure Alzheimer's Fund has been able to support some of the best scientific minds in the field of Alzheimer's research. For more information, please visit <http://www.curealz.org/>.

View source version on [businesswire.com](https://www.businesswire.com/news/home/20201109005617/en/): <https://www.businesswire.com/news/home/20201109005617/en/>

**Media Contact:**

Merissa Muller  
Finn Partners  
(617) 778-6633  
[Merissa.Muller@finnpartners.com](mailto:Merissa.Muller@finnpartners.com)

Source: Amylyx Pharmaceuticals, Inc.