



## **Amylyx Pharmaceuticals Announces AMX0035 Demonstrated Statistically Significant Treatment Benefit for People with ALS in the CENTAUR Trial**

### ***Study Meets Primary Endpoint of Slowing of ALS as Measured by the ALSFRS-R***

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CAMBRIDGE, Mass.--(BUSINESS WIRE)--Amylyx Pharmaceuticals, Inc., a pharmaceutical company focused on developing new treatments for amyotrophic lateral sclerosis (ALS) and other neurodegenerative diseases, and the Sean M. Healey & AMG Center for ALS at Mass General today announced that AMX0035 demonstrated a significant treatment benefit for people with ALS in the CENTAUR study. In the study, participants taking AMX0035 had a statistically significant slowing of ALS disease progression as measured by the ALS Functional Rating Scale-Revised (ALSFRS-R) compared to placebo ( $p < 0.05$ ), the primary outcome of the trial.

Detailed results from CENTAUR will be submitted for peer-reviewed publication and presentation at a future medical congress.

"Today marks a significant step forward in the fight to develop new treatments for ALS," said Dr. Sabrina Paganoni, M.D., Ph.D., principal investigator of the CENTAUR study, investigator at the Healey Center for ALS at Mass General and Assistant Professor of PM&R at Harvard Medical School and Spaulding Rehabilitation Hospital. "The study results highlight AMX0035 as a potentially beneficial new treatment for people with ALS, and the design and execution of the CENTAUR trial are a testament to true collaboration across the many stakeholders in this fight. Thank you to everyone who made this possible, and I look forward to presenting the full study results in the coming months."

"We are honored and humbled to have reached this milestone after working nearly seven years on the development of AMX0035," said Joshua Cohen, CEO, Chairman, and Co-Founder of Amylyx. "Thank you to each and every participant, family, physician, nurse, coordinator, vendor, and advisor who has and continues to work with us to better the lives of people with ALS."

“With these results, Amylyx now has a responsibility to move ahead as efficiently as possible, as people living with ALS don’t have time to wait,” said Justin Klee, President and Co-Founder of Amylyx. “We will work closely with the FDA and the ALS community, including the wonderful Northeast ALS Consortium leadership and member sites that conducted the CENTAUR trial, to decide next steps and will continue to keep everyone informed.”

Participants in CENTAUR were given the option after the trial to enroll in an open-label extension study to receive treatment with AMX0035. Nearly 90 percent of participants who completed CENTAUR elected to enroll in the extension study. Interim data from the ongoing extension study will be presented in 2020.

In addition, the company will provide an update on regulatory plans and further details on expanded access plans in early 2020.

“ALS Finding a Cure is proud to have catalyzed and supported the CENTAUR study, and I am encouraged by what the results mean for people living with ALS. Our team at the Mass General Neurological Clinical Research Institute is proud of this collaboration with Amylyx and our colleagues in the Northeast ALS Consortium on this important study,” commented Dr. Merit Cudkowicz, Chief Medical Officer from ALS Finding a Cure®, Director of the Healey Center for ALS, Chief of Neurology at Mass General, and the Julieanne Dorn Professor of Neurology at Harvard Medical School. “We look forward to advancing this research and what it might mean for those living with ALS.”

Dr. Neil Thakur, executive vice president for mission strategy at The ALS Association, added, “We are proud to have supported AMX0035 and Amylyx from an early stage and are very excited about what AMX0035 may accomplish for people with ALS. This company and study team have focused on the patient perspective during the design and conduct of this study and we are happy to work with and innovate together with them. We are excited to continue to collaborate on this therapy in the future.”

Dr. Rudolph Tanzi, Ph.D., Kennedy Professor of Neurology, Massachusetts General Hospital, chair of the Cure Alzheimer’s Fund Research Leadership Group and chair of the Amylyx SAB, shared, “The positive results from the CENTAUR ALS study demonstrate that the novel mechanism of AMX0035 may represent a new treatment approach for not only ALS, but for Alzheimer’s disease. I am very excited about the demonstrated benefit of AMX0035 in people with ALS, and look forward to the results from the ongoing PEGASUS trial for people with Alzheimer’s disease.”

ALS, also known as Lou Gehrig’s disease, is a progressive neurodegenerative disease that affects nerve cells in the brain and the spinal cord. The progressive neurodegeneration in ALS eventually leads to the death of motor neurons and loss of the ability of the brain to initiate and control muscle movement. With muscle action progressively affected, patients in the later stages of the disease may become paralyzed and unable to breathe normally.

### **About AMX0035**

AMX0035 is Amylyx’ first in class investigational therapy designed to reduce neuronal death and dysfunction. AMX0035 targets mitochondrial and endoplasmic reticulum dependent neuronal degeneration pathways.

### **About CENTAUR**

CENTAUR was a 24-week, randomized, double-blind, placebo-controlled Phase II clinical trial that evaluated the safety and tolerability of AMX0035 and assessed the drug’s impact on disease progression as measured by the revised Amyotrophic Lateral Sclerosis Functional Rating Scale (ALSFRS-R) when compared to placebo.

The trial also assessed the effects of AMX0035 on other measures that are critical to people with ALS, including muscle strength, lung vital capacity, and biomarkers of neuronal degeneration.

CENTAUR enrolled patients 18-80 years old with definite ALS and within 18 months of symptom onset. The trial did not restrict patients from receiving edaravone or riluzole. The CENTAUR study was conducted by investigators at 25 top ALS medical centers through the Northeast ALS (NEALS) consortium. NEALS is an international, independent, non-profit group of researchers who collaboratively conduct clinical research in Amyotrophic Lateral Sclerosis (ALS) and other motor neuron diseases. NEALS researchers are dedicated to finding treatments for people with ALS and motor neuron disease as rapidly as possible.

More information on the CENTAUR trial can be found at <http://amylyx.com/trials/> or [www.clinicaltrials.gov](http://www.clinicaltrials.gov), NCT03127514.

In addition, there is an open label extension study (CENTAUR-OLE) allowing enrolled participants to continue to receive AMX0035. More information on the open label extension can be found at <http://amylyx.com/trials/> or [www.clinicaltrials.gov](http://www.clinicaltrials.gov), NCT03488524.

CENTAUR was the recipient of the ALS ACT grant, and is supported by [ALS Finding a Cure®](#), -a program of The Leandro P. Rizzuto Foundation, [The ALS Association](#), the [Northeast ALS Consortium](#), [Mass General Neurology Clinical Research Institute](#), and was funded in part by the ALS Ice Bucket Challenge.

### **About Amylyx Pharmaceuticals**

Amylyx Pharmaceuticals, Inc. is a pharmaceutical company 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](#).

### **About the Sean M. Healey & AMG Center for ALS at Mass General**

The Sean M. Healey & AMG Center for ALS at Mass General includes a diverse team of researchers, clinicians, project managers, and information technologists who are determined to accelerate the pace of ALS therapy development. Under the leadership of Merit Cudkowicz, MD, and a Science Advisory Council of international experts, Healey faculty partner with scientists, clinicians, and people with ALS from all over the world in a shared mission to develop treatments to cure ALS. For more information, visit [www.massgeneral.org/neurology/als](http://www.massgeneral.org/neurology/als) and follow us on [Twitter](#).

### **About The ALS Association**

The ALS Association is the largest philanthropic funder of ALS research in the world. The Association funds global research collaborations, provides assistance for people with ALS and their families through its nationwide network of chapters and certified clinical care centers, and advocates for better public policies for people with ALS. The ALS Association builds hope and enhances quality of life while urgently searching for new treatments and a cure. For more information about The ALS Association, visit our website at [www.alsa.org](http://www.alsa.org).

### **About ALS Finding a Cure**

ALS Finding a Cure® is a research organization dedicated to being a game changer in discovering a cure for this fatal disease. Founded in 2014 by noted philanthropist and Conair Founder and Chairman, Leandro (Lee) Rizzuto, as a tribute to Christie Rizzuto, Lee's daughter-in-law who was diagnosed with ALS in 2009 at the age of 41, and under the joint leadership of Denis Rizzuto and Peter N. Foss, ALS Finding a Cure® is focused on

identifying the gaps in the scientific understanding of ALS that are preventing the development of a cure. The organization, a program of The Leandro P. Rizzuto Foundation, collaborates with a wide range of companies, ALS organizations, other disease non-profits, and ALS patients and families to ensure research efforts are non-duplicative, synergistic, and focused on the ultimate goal: getting to a cure. For more information about ALS Finding a Cure®, visit [www.alsfindingacure.org](http://www.alsfindingacure.org).

### **About the Northeast ALS Consortium**

The [Northeast ALS Consortium](#) (NEALS) is an international, independent, non-profit group of 128 research sites around the world who collaboratively conduct clinical research in Amyotrophic Lateral Sclerosis (ALS) and other motor neuron diseases. The mission of NEALS is to translate scientific advances into new treatments for people with ALS and motor neuron disease as rapidly as possible. The NEALS network is governed by an Executive Committee and the Consortium research activities are advised by an experienced Scientific Advisory Board. Each Leadership team member brings a depth of experience and a unique perspective to the governance of NEALS. Through this leadership, active oversight is provided for ongoing operations along with new approaches to trial design evaluated and implemented.

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