



Amylyx Pharmaceuticals to Present Data from First-in-Human, Dose-Ranging Phase 1 LUMINA Trial of AMX0114 in ALS at ENCALS 2026

June 23, 2026

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jun. 23, 2026-- [Amylyx Pharmaceuticals, Inc.](#) (NASDAQ: AMLX) (“Amylyx” or the “Company”) today announced the presentation of data from its first-in-human, dose-ranging Phase 1 LUMINA trial of AMX0114 at the European Network to Cure ALS (ENCALS) Meeting 2026, held from June 24-26, 2026 in Madrid, Spain.

AMX0114 showed no drug-related serious adverse events (SAEs) and no serious neurological adverse events (AEs) in Cohort 1 (lowest dose level 12.5 mg). These safety findings support continued evaluation of AMX0114 at higher dose levels in this multiple ascending dose trial. Biomarker levels, including spectrin breakdown product 145 (SBDP-145), neurofilament light (NfL), and phosphorylated neurofilament heavy (pNFH), remained near baseline. Cohort 1 evaluated the lowest dose level, and future cohorts will evaluate the impact of higher dosage levels.

LUMINA ([NCT06665165](#)) is a multinational, randomized, double-blind, placebo-controlled, multiple ascending dose clinical trial of AMX0114, an investigational, antisense oligonucleotide (ASO) targeting calpain-2, in people living with amyotrophic lateral sclerosis (ALS). LUMINA is evaluating the safety, tolerability, pharmacokinetics, and pharmacodynamics along with ALS biomarkers. LUMINA study sites are active in Canada and the United States. Cohorts 1 (12.5 mg) and 2 (25 mg) are fully enrolled, and Cohort 3 (50 mg) is currently enrolling. The Company anticipates a total of four dose levels in this dose-ranging study.

The ENCALS 2026 poster presentation details are as follows:

Title: Advancing AMX0114 for ALS: Updates from the LUMINA Phase 1 Study

Presenter: Lauren Kett, MD, PhD

Poster Number: PO119

Date and Time: Wednesday, June 24, 2026, 5:20 – 7:00 p.m. CEST

For more information about the ENCALS Meeting 2026, please visit <https://www.encals.eu/>. The presentation will be available on the “Events & Presentations” tab of the Amylyx website.

About AMX0114

AMX0114 is an investigational antisense oligonucleotide (ASO) with U.S. Food and Drug Administration (FDA) Fast Track designation for the potential treatment of ALS. AMX0114 targets calpain-2 (*CAPN2*), a calcium-activated protease that is one of the fundamental drivers of axonal degeneration and consequent disease progression in ALS. In preclinical studies, treatment with AMX0114 resulted in potent, dose-dependent, and durable reduction in *CAPN2* mRNA and calpain-2 protein levels in disease-relevant cell models of axonal degeneration. This translated to improved neuronal survival, including in a model of TDP-43 ALS, and reductions in extracellular neurofilament light (NfL) levels across multiple disease models and paradigms of neuronal injury. AMX0114 was generally well-tolerated in LUMINA trial participants enrolled in Cohort 1 (n=12), with no treatment-related serious adverse events (SAEs).

About LUMINA

The Phase 1 LUMINA clinical trial ([NCT06665165](#)) is a multinational, randomized, double-blind, placebo-controlled, multiple ascending dose trial evaluating the safety, tolerability, pharmacokinetics, and pharmacodynamics of AMX0114 across four dose levels in people living with ALS. LUMINA will also assess change from baseline in calpain-2 levels, neurofilament light (NfL) levels, and other pharmacodynamic biomarkers of ALS. LUMINA is anticipated to enroll approximately 48 adult participants. Participants will be randomized 3:1 to receive AMX0114 or placebo by intrathecal administration once every four weeks for a total of up to 4 doses. Study sites are active in Canada and the United States. Cohorts 1 and 2 have been fully enrolled, and the Company is currently enrolling Cohort 3.

About Amylyx Pharmaceuticals

At Amylyx, our mission is to usher in a new era of treating diseases with high unmet needs. Where others see challenges, we see opportunities that we pursue with urgency, rigorous science, and unwavering commitment to the communities we serve. We are currently focused on four investigational therapies across several endocrine conditions and neurodegenerative diseases in which we believe can make the greatest impact. For more information, visit amylyx.com and follow us on [LinkedIn](#) and [X](#). For investors, please visit investors.amylyx.com.

Forward-Looking Statements

Statements contained in this press release regarding matters that are not historical facts are “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995, as amended. Because such statements are subject to risks and uncertainties, actual results may differ materially from those expressed or implied by such forward-looking statements. Such statements include, but are not limited to, Amylyx’s expectations regarding: the potential for AMX0114 as a treatment for ALS, the expected timeline for enrollment of future cohorts, and the expected timeline for data readout. Any forward-looking statements in this press release and related comments in the Company’s earnings conference call are based on management’s current expectations of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in or implied by such forward-looking statements. Risks that contribute to the uncertain nature of the forward-looking statements include: the success, cost, and timing of Amylyx’s program development activities; Amylyx’s ability to execute on its regulatory development plans and expectations regarding the timing of results from its planned data announcements and initiation of clinical studies; Amylyx’s ability to fund operations, and the impact that global macroeconomic uncertainty, geopolitical instability, and public health events will have on Amylyx’s operations, as well as the risks and uncertainties set forth in Amylyx’s United States Securities and Exchange Commission (SEC) filings, including Amylyx’s Annual Report on Form 10-K for the year ended December 31, 2025, and subsequent filings with the SEC. All forward-looking statements contained in this press release and related comments in our earnings conference call speak only as of the date on which they were made. Amylyx undertakes no obligation to update such statements to reflect events that occur or circumstances that exist after the date on which they were made, except as required by law.

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