Amylyx Pharmaceuticals Announces Donated CENTAUR Clinical Trial Data Now Available to Help Advance Science in ALS for Future Treatments and Discoveries

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CAMBRIDGE, Mass.--(BUSINESS WIRE)--Sep. 14, 2022-- Amylyx Pharmaceuticals, Inc. today announced that the donated clinical data from the placebo arm of the CENTAUR clinical trial that evaluated the safety, efficacy, and survival benefits of AMX0035 in adult participants with amyotrophic lateral sclerosis (ALS) are now available in the Pooled Resource Open-Access ALS Clinical Trials (PRO-ACT) database. The PRO-ACT project is led by Alex Sherman at the Neurological Clinical Research Institute (NCRI) at Mass General Hospital and is currently sponsored by The ALS Association.

“The only way we are going to find a cure for ALS is if the entire community shares data and builds upon the findings of others—that is how we can continue to move forward and develop innovative therapies to end ALS,” said Joshua Cohen and Justin Klee, Co-CEOs of Amylyx. “Continually adding to the database will give the ALS research community new information that could open up new pathways and approaches to developing new treatments. This platform also serves as an invaluable resource to analyze and validate the robustness of research data sets.”

The PRO-ACT database houses the largest ALS clinical trials dataset, containing more than 11,600 anonymized ALS participant records from 28 completed clinical trials. The platform harmonizes and merges data from existing publicly and privately conducted ALS clinical trials to generate a unique, freely available resource for the scientific community to find cures for ALS. The PRO-ACT platform was selected as the Bio-IT World’s Best Practices Awards winner in 2013 and The Clinical Informatics News Best Practices winner in the Clinical Data Intelligence category in 2016. In 2021, PRO-ACT won a prestigious Healey International Prize for Innovation in ALS for “enabling the new field of ALS predictive analytics.”

"The PRO-ACT database enables the type of collaboration necessary to accelerate the search for treatments and, ultimately, cures for ALS,” said Kuldip Dave, Ph.D., Senior Vice President of Research at the ALS Association. “By pooling resources and sharing data with researchers around the world, we move closer to creating a world without ALS.”

The PRO-ACT platform was created by Prize4Life, a non-profit organization, in partnership with the NEALS Consortium and the NCRI at Mass General Hospital, with funding from The ALS Therapy Alliance, Prize4Life, NCRI, and The ALS Association. To date, PRO-ACT has served as the primary data source for more than 70 publications and has been critical for numerous others. The platform allows researchers to better understand disease heterogeneity and develop novel predictive, staging, and survival models of the disease. It has been a critical tool to support the design of multiple ALS clinical trials.

“The continued addition of data to the PRO-ACT platform is what makes it a valuable tool to the ALS community,” said Avi Kremer, Founder of Prize4Life, an ALS Association consultant and a person living with ALS. “Adding data that takes into account newer approaches to ALS treatment allows the community to build on past work and move us closer to a cure.”

“Letting data sit behind locked doors of a single entity can stifle research by the broader community,” said Alex Sherman, Director of the Center for Innovation and Biomedical Informatics (CIB) at NCRI and Healey & AMG Center at Mass General, Principal Associate in Neurology at Harvard Medical School, leader of the team that created the PRO-ACT database, and the current Principal Investigator of the PRO-ACT platform. “Sharing of data with the broader community allows others to look at things from a different point of view and potentially unlock something that others had missed. We hope companies continue to see the value in sharing data and follow Amylyx in data donations.”

About AMX0035
AMX0035 (sodium phenylbutyrate and taurursodiol) is an oral fixed-dose medication approved with conditions as ALBRIOZA™ to treat amyotrophic lateral sclerosis (ALS) in Canada and with marketing applications pending in the United States and European Union. The combination of sodium phenylbutyrate and taurursodiol may reduce neuronal cell death, hypothesized to occur by simultaneously mitigating endoplasmic reticulum (ER) stress and mitochondrial dysfunction. AMX0035 is also being explored for the potential treatment of other neurodegenerative diseases.

About the CENTAUR Trial
CENTAUR was a multicenter Phase 2 clinical trial in 137 participants with ALS encompassing a 6-month randomized placebo-controlled phase and an open-label long-term follow-up phase. The trial met its primary efficacy endpoint of reducing functional decline as measured by the Amyotrophic Lateral Sclerosis Functional Rating Scale – Revised (ALSFRS-R).

Overall, reported rates of adverse events and discontinuations were similar between AMX0035 and placebo groups during the 24-week randomized phase; however, gastrointestinal events occurred with greater frequency (≥2%) in the AMX0035 group. Detailed safety and functional efficacy data from CENTAUR were published in the *New England Journal of Medicine*. Data from additional analyses from the CENTAUR trial were published in *Muscle & Nerve* in 2020 and 2022, and the Journal of *Neurology, Neurosurgery and Psychiatry* in 2022.

The CENTAUR trial was funded, in part, by the ALS ACT grant and the ALS Ice Bucket Challenge, and was supported by The ALS Association, ALS Finding a Cure (a program of The Leandro P. Rizzuto Foundation), the Northeast ALS Consortium, and the Sean M. Healey & AMG Center for ALS at Mass General.

About Neurological Clinical Research Institute at Mass General Hospital
The Neurological Clinical Research Institute (NCRI) at Massachusetts General Hospital accelerates translational research in neurological disorders through initiating and testing novel therapies. The NCRI has an extensive history in leading clinical research to find new treatments for neurological diseases including Amyotrophic Lateral Sclerosis (ALS), myasthenia gravis, diabetic neuropathy, stroke, multiple sclerosis, Parkinson’s disease, and Huntington’s disease. For more information, visit www.NCRInstitute.org or www.data4cures.org.

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 our nationwide network of care and certified clinical care centers, and advocates for better public policies for people with ALS. The ALS Association is working to make ALS a livable disease while urgently searching for new treatments and a cure. For more information about The ALS Association, visit our website at www.als.org.

About Amylyx Pharmaceuticals
Amylyx Pharmaceuticals, Inc. is committed to supporting and creating more moments for the neurodegenerative community through the discovery and development of innovative new treatments. Amylyx is headquartered in Cambridge, Massachusetts and has operations in Canada and EMEA. For more information, visit amylyx.com and follow us on LinkedIn and Twitter. 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, statements regarding the potential of AMX0035 or other future therapeutic candidates as a treatment for ALS and neurodegenerative diseases generally and the benefits of data sharing to develop new treatments and/or cure ALS. Any forward-looking statements in this press release 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’ program development activities, Amylyx’ ability to execute on its strategy, regulatory developments, expectations regarding the timing of regulatory review of AMX0035 and the inherent unpredictability of the regulatory review process, Amylyx’ ability to fund operations, and the impact that the ongoing COVID-19 pandemic will have on Amylyx’ operations, as well as those risks and uncertainties set forth in Amylyx’ United States Securities and Exchange Commission (SEC) filings, including Amylyx’ Annual Report on Form 10-K for the year ended December 31, 2021, and subsequent filings with the SEC. All forward-looking statements contained in this press release 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.

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