Amylyx is a late preclinical stage pharmaceutical company dedicated to providing disease-modifying solutions to neurodegenerative diseases. Our highly creative and innovative team is focused on rapid and innovative clinical development.

**Company Overview**

Amylyx incorporated in 2013 and is a privately held Delaware C Corporation operating in Cambridge, Massachusetts. Amylyx was founded on the fundamental notion that the treatment of complex, chronic diseases will require broad pathway understanding and more than one target and/or therapeutic. With this premise, we expanded upon the Amyloid Cascade Hypothesis and developed a more holistic theory that presents Alzheimer’s disease (AD) as a complex, multi-pathway cycle rather than a linear progression. We then selected two safe, pre-existing compounds that together would synergistically halt the pathologies at their checkpoints. Excited by the new angle of treatment, Dr. Rudolph Tanzi, one of the ten most cited Alzheimer’s Disease (AD) researchers and the co-discoverer of the three early onset Alzheimer’s disease genes, the Huntington’s gene, and the first-discovered ALS gene, joined Amylyx as a founder and Chairman of our Scientific Advisory Board. We have subsequently brought together a distinguished team of advisors and consultants with a variety of backgrounds and experiences. We intend to bring our therapeutic to the clinic as fast as possible so as to start treating the millions of patients currently suffering from neurodegenerative diseases.

The Amyloid Cascade Hypothesis postulates that overproduction of amyloid beta results in a myriad of pathologies including inflammation, hyperphosphorylation of tau protein, synaptic tangling, and cell death. While volumes of studies both academic and clinical have expanded the theory that amyloid is the only and root cause of the disease, drug candidates targeting the Cascade have failed to meet endpoints. Recent biomarker and imaging studies in patients have found that brain atrophy, which amyloid drugs do not slow, is the strongest correlate to and maybe a major cause of clinical decline. Furthermore, neuro-inflammation has recently been shown to be a major accelerant of pathology and is also not blocked by amyloid-targeting drug candidates. We therefore designed a disease mechanism and treatment that would account for these integral gaps in the hypothesis. Examining other neurodegenerative fields, we determined that neuronal death and inflammation are also major missing targets in the treatment of ALS and Parkinson’s (PD).

Our lead therapeutic candidate, AMX0035, is designed to halt the UPR and mitochondrial stress pathways. By simultaneously targeting both pathways, we have dramatically protected neurons from dying, even under the aggressive environments in AD, ALS, or PD. Additionally, results from an *in vitro* immunological model showed that AMX0035 is strongly immune-modulatory. In a transgenic mouse model of AD, our therapeutic improved cognition and, to our surprise, significantly reduced amyloid. We are currently running a large *in vivo* study with the Prize4Life foundation to test our candidate in Amyotrophic Lateral Sclerosis (ALS). We are designing and testing AMX0035 in a novel, *in vivo* neuro-inflammatory model, as recommended by a potential pharmaceutical partner, as well as planning to test in an established *in vivo* model of Parkinson’s disease.

Amylyx has completed a convertible note seed-financing round and has received non-dilutive support from several organizations, including Brown University and the Prize4Life Foundation. Amylyx is now completing the planning of all necessary steps to develop our therapeutic candidate into a clinic-ready product; we have received quotes for all steps of IND-enablement, built relationships in all relevant industries, and have spoken with many industry leaders in neurological drug development. We have acquired the right to cross-reference to an investigational new drug application for one of our components and the other is FDA approved streamlining our path to clinic. We are determined to fund and complete all IND-enabling studies in order to treat our first patient by Q1 2016.

Thank you and we hope you may join us in our fight against these devastating diseases.