Les Turner ALS Foundation
5550 W. Touhy Avenue, Suite 302
Skokie, IL 60077-3254

847 679 3311
lesturnerals.org

March 16, 2022

RE: Docket No. FDA-2018-N-0410

Dear FDA Advisory Committee:

Founded in 1977, the Les Turner ALS Foundation is the oldest independent ALS group in the country. For 45 years, it has been our mission to provide the most comprehensive care and support to people living with ALS and their families so they can confidently navigate the disease and advance scientific research for the prevention, treatment and cure of ALS. We are committed to providing access to the most promising therapies for people living with this devastating disease. **We have no time to waste.**

ALS is a terminal and progressive disease that causes muscle weakness, difficulty speaking and swallowing and, generally, complete paralysis. ALS can, in some cases, also cause changes in intellectual function, mood, behavior or personality. While technically considered a "rare disease," experts predict an individual's lifetimes risk of acquiring ALS is about 1 in 300 by the age of 85. There is no cure, yet.

In the 45 years of the existence of the Les Turner ALS Foundation, only two drugs have been approved by the FDA to treat ALS. We desperately need more options to treat ALS. We have **no time to waste to approve AMX0035.**

AMX0035 is the first ALS therapeutic to demonstrate both a statistically significant survival and functional benefit in ALS. There is no safety signal in AMX0035, the adequacy and clinical meaningfulness of the data was clearly demonstrated in a well-designed, robust and randomized controlled trial at 25 top clinical trial sites in the United States. **There is an urgent need for access to safe and effective therapies and regulatory flexibility for unmet medical needs. We have no time to waste.**

ALS is scary. Really scary. For people living with ALS and their caregivers, life has become so tumultuous that they often feel exhausted, overwhelmed and hungry for emotional comfort and effective treatments.

Since no two people with ALS are alike or will progress the same way, our Les Turner ALS Foundation ALS Support Services Coordinators work with people with ALS on a personalized basis to manage the rapid progression, psychosocial needs and constant losses of function. We do this by working in close partnership with clinicians at the Lois Insolia ALS Clinic at the Les Turner ALS Center at Northwestern Medicine. We help them adjust to speech changes, help them and their families make decisions about nutrition and breathing support, and help them with the constant flow of new equipment to ensure safety in the home.

Each year, we lose about one-third of the people we directly serve to ALS. This month alone, we tragically lost an 18 year-old boy in a few short months to a very rapid form of ALS. Despite our 45 years of experience, we were not able to keep up with the progression of this disease. AMX0035 has been shown to slow ALS disease progression by two points on the ALSFRS-R scale and extend life by 6.5 months. These numbers may not sound like much, but for a disease that takes lives within an average of 2-5 years, this slowing of progression extends the time the families who face this life-threatening disease have together. Slower disease progression is meaningful to each person living with ALS and their loved ones.

Slowing the disease progression means more graduations, more weddings and more family holidays. Slowing the disease progression means cutting up your own food, speaking on your own and even breathing on your own. Slowing the disease progression means more handwritten letters to loved ones, more climbs up the stairs to sleep in your room and simply more time with those you love. **We have no time to waste.**

We urge the FDA advisory committee to recommend full approval of AMX0035. We have no time to waste.

Sincerely,

Andrea Pauls Backman, MBA
Chief Executive Officer

Disclosure: As a non-profit, we receive less than 2% of our annual funding from Amylyx Pharmaceuticals, Inc. as well as other pharmaceutical companies.