AMX0035 FAQ’s

Amylyx, a Massachusetts-based pharmaceutical company, published the results of a multi-center placebo-controlled double-blind phase 2 trial of its compound AMX0035 on September 3 in the New England Journal of Medicine. The results of the trial were very promising – people with ALS receiving AMX0035 experienced a significantly slower decline in disease progression, compared to those on a placebo. Additionally, AMX0035 was safe and well-tolerated indicating a good benefit/risk consideration for people with ALS.

What is The ALS Association’s reaction to this news?

We are optimistic that AMX0035 can help people with ALS. We believe the data makes a clear and compelling case that it should be made available to people with ALS as soon as possible. We look forward to working with Amylyx, the FDA, and the ALS community to make that happen.

What were the results of AMX0035 on participants in the clinical trial?

Clinical trial participants who received AMX0035 experienced a clinical meaningful delay in ALS progression as measured by the Amyotrophic Lateral Sclerosis Functional Rating Scale (ALSFRS). For many trial participants, that delay meant the difference between being able to feed oneself versus being fed or needing, versus not needing a wheelchair.

How does AMX0035 work?

AMX0035 is a combination of two existing drugs, sodium phenylbutyrate and taurursodiol, which acts to prevent nerve cell death by blocking stress signals in cells. Unlike other treatments in development, AMX0035 does not target the root cause of ALS. Instead, it aims to preserve the motor neurons that are progressively lost in ALS patients, slowing clinical decline.

How is AMX0035 administered?

AMX0035 is an oral therapy (a suspension in water, taken twice daily by swallowing or via PEG tube).

How many people were in enrolled in the trial and across how many sites?

The trial included 137 people with ALS and was conducted across 25 top medical centers through the Northeast ALS (NEALS) consortium.

What were the eligibility requirements for the trial?

All individuals enrolled in the trial were diagnosed with sporadic or familial ALS within the previous eight months, and all had rapidly progressing disease. These are stringent enrollment criteria meant to provide the clearest information possible.

When can people with ALS access AMX-0035?

In a best case scenario, Amylyx will submit a new drug application (NDA) to the FDA before the end of the year. FDA will take several months to review the NDA and can then use its authority to waive the phase 3 clinical trial to expedite AMX-0035 to market, where additional “real world” study of the drug can continue. This process would likely follow a 12 to 15-month timeline. The other scenario would require the same process plus a three-year phase 4 trial. Given that the drug has already been well tolerated and is shown to be effective in slowing disease progression, we don’t believe the delay and additional information gained from a phase 3 trial is needed. Through our petition initiative with I AM ALS, we are urging FDA and Amylyx to work together to bring the drug to market as soon as possible, without a phase 3 trial. To sign the petition, visit: www.als.org/petition.

What’s next for AMX0035?

Urgent action is necessary. People with ALS cannot wait for the full experimental process for AMX0035 to continue. We are asking the FDA and Amylyx to work together to bring AMX0035 to people with ALS as soon as possible, prior to a phase 3 trial. We ask that the FDA conduct a swift review, require aggressive follow-on studies, and that Amylyx allows expanded access of the AMX0035 until approval.

Was this trial funded by The ALS Association?

The ALS Association supported this trial through ALS Ice Bucket Challenge donations, with $2.2 million in grants toward the company and the clinical trial network. We did this in partnership with ALS Finding a Cure for a total of $2.96 million.
Palliative Care vs. Hospice Care
By: Melissa Newby, RN and Jessica Higgins, RN at Phoenix Home Care and Hospice

What is the difference between hospice and palliative care?
Palliative Care is a program that provides resources for advanced care planning to persons who have a life-threatening illness and are seeking aggressive treatment. Palliative care takes a holistic approach focusing on the whole person with use of resources to assist in navigating and improving communication for the best care of the patient and family. The palliative care team uses their expertise to advocate for medical care and personal choices eliminating gaps in care or service failures. The palliative care team will coordinate with other medical professionals such as physicians, clinics, home health, and hospice to ensure and maximize quality of life and patient choice.

Hospice care is for individuals with a terminal illness who have decided to no longer pursue aggressive treatment and instead choose quality of life. Hospice is hands-on medical care for the patient and provides education, resources and support for caregivers. Hospice has 24/7 on call nursing services, provides medical equipment, supplies, bathing services, volunteers, chaplain, and social work services.

Does insurance cover hospice and palliative care?
Hospice is 100 percent covered under the Medicare benefit and most insurances have a hospice benefit. Palliative care is no charge to the patient and family.

How do I know which is the best option for me?
Palliative care allows continuation of aggressive treatments and can prepare you for hospice services. Hospice services are for those that have decided to stop aggressive treatment and choose quality of life.

What is covered under the hospice Medicare benefit?
Medical equipment, supplies such as briefs/wipes/gloves/needed dressings, medications, nursing services, home health aide services, social work services, Chaplain services, bereavement services, volunteers, and respite care.

For more information about hospice and palliative care, contact your local ALS Navigator.

Walk Your Way
The Kansas City Walk to Defeat ALS draws nearly 3,000 participants and raises in excess of $600,000 each year. The Walk is our largest and mostly widely attended event each year and provides nearly 50 percent of our funding for the year, so we can continue providing valuable services to those facing ALS free-of-charge. Although we are disappointed that we cannot all walk together this year at Kauffman Stadium, ALS doesn’t stop in a pandemic, neither do we.

This year, we are asking everyone to Walk Your Way on October 3rd, 2020! There are so many ways to Walk Your Way, but the most important thing is to unite, to raise funds and awareness for ALS. Below are a few examples of how to Walk Your Way.

1. Get outside and walk around your neighborhood, wearing your team shirts.
2. Start a car caravan! Honk if you love someone who is living with ALS.
3. Take a ton of photos and videos and share with us on our social media pages.

We will have the opening ceremony live on the Chapter’s Facebook page (The ALS Association Mid-America Chapter) at 10:00 am. The ceremony will include a ribbon cutting and awards for the Top Team and Top Fundraiser. You won’t want to miss out on all of the fun! There will also be check-ins and updates throughout the day.

Don’t want to walk? No problem! Go play golf, swing on your porch or sit outside. The idea is to do something, anything, to raise funds and awareness on October 3rd! Can’t do October 3rd? Not a problem either! Pick a day that works for you and your family and walk then. That’s the great thing about this year- you decide where, when and how. We are here to help you every step of the way as you plan what Walking Your Way will look like!

Be sure to share your efforts on social media so we can follow along on all of the fun! Tag @ALSMidAmerica on Facebook and use the hashtag #WalkYourWayKC to join the movement. Also be sure to “like” The Kansas City Walk to Defeat ALS Facebook page to stay up-to-date on all things Walk!

If you haven’t registered a team for the Kansas City Walk to Defeat ALS yet, there is still time! It’s free to register for the event and there is no team size requirement- you can even walk just you! For more information on how to get started, reach out to Erin McNamara at emcnamara@alsa-midamerica.org or 913-648-2062 ext. 207. You can also visit our website at http://web.alsa.org/KansasCity.

Virtual Grief Support Group
Feeling isolated due to social distancing? Want to stay connected with others experiencing loss? Looking for a safe space to share your story? Join us on Zoom on Wednesday, October 21st at 6:30 p.m. for a virtual grief support group.

The ALS Association Mid-America Chapter and Ascend Hospice have partnered together to provide a virtual grief support group. The group will meet every third Wednesday of the month, from 6:30 p.m. to 8 p.m., for those who have lost a loved one to ALS. With the need for social distancing, this group will allow us to gather together to share our unique stories and support each other as we grieve.

For directions on how to join a virtual meeting, please contact Cheri Ball (Ascend Hospice Bereavement Coordinator) at 816-506-5763 or email Cheri at cheri.ball@ascendhealth.com.

ALS Focus Survey
ALS Focus is a patient and caregiver-led survey program that asks people impacted by ALS about their needs and burdens. The goal is to learn about individual experiences throughout the disease journey so that the entire ALS community can benefit.

Data and findings collected from the surveys are de-identified. The data will be used to inform decisions and strengthen programs and policies around drug development, clinical trial design, regulatory review, drug payment and reimbursement, clinical care, home health, and more.

Registering for Focus and completing the surveys will take approximately 15-25 minutes. For more information, and to access the survey, visit: www.als.org/als-focus.