

'If not for the Ice Bucket Challenge, we wouldn't be here': Company on the brink of FDA-approved ALS treatment

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Credit: Pixabay/CC0 Public Domain

Eight years after the ALS Ice Bucket Challenge went viral across social media and raised hundreds of millions of dollars for research, a

Cambridge company is on the brink of a major breakthrough for ALS patients.

Amylyx Pharmaceuticals should receive word in the coming week from the U.S. Food and Drug Administration on whether the feds will approve the local firm's drug treatment for the relentlessly progressive and fatal neurodegenerative disorder Amyotrophic Lateral Sclerosis, or ALS.

AMX0035, which has already been approved in Canada, is the first treatment funded by ALS Ice Bucket Challenge donations that's being considered for approval by the FDA.

"If not for the Ice Bucket Challenge, we wouldn't be here," Justin Klee, co-CEO and co-founder of Amylyx, told the Herald.

If approved by the FDA, Amylyx's drug would be a significant development for the ALS community, which has few treatment options. About 30,000 people in the U.S. have ALS, according to estimates.

The company's drug in trials showed that it can slow the disease's progression and help patients survive longer.

"It's the first time we've seen a benefit on both function and survival in the same drug in the same trial, which is I think why people are really excited," Klee said.

Data from the company's trial of AMX0035 showed that the drug slowed down [disease progression](#) by at least 25%, and increased lifespan by several months.

"Our trial showed a statistically significant slowing in the rate of disease progression," said Josh Cohen, co-CEO and co-founder of Amylyx. "People progressed about 25% slower when they were on the drug as

compared to placebo.

"We also saw that people who were on the drug survived longer than those who were taking the placebo, as well," he added. "We saw a benefit on both function and survival."

Cohen emphasized that their drug is not a cure.

"ALS is still not a disease anybody would ever want to get, even with this [drug](#), but what it does is it makes the disease a little bit slower and kind of gives people more time to be functional and ideally more time to survive, as well," he said.

A decade ago, when Cohen and Klee were recent college graduates launching the company, they were struggling to receive financial investments in the firm. Then local ALS patient Pete Frates, along with ALS patient Pat Quinn and their families started the Ice Bucket Challenge.

"And all of a sudden, there was funding in ALS, where previously there had been very little," Klee said.

Some of that funding went to grants, and Amylyx received a \$3 million grant. With that, the company was able to raise significant funding.

"Now all these years later, it looks like this might actually be a treatment for people with ALS, and a really important one," Klee said.

The company also has a personal connection to the Frates family. Frates' cousin Jim is Amylyx's CFO.

He said it has been "incredibly moving" being part of the company helping to treat ALS.

"The people living with ALS, their caregivers and all the doctors and researchers in the field are just so inspiring," he said. "The dignity and determination they show every day really inspires all of us at Amylyx to do our best to deliver for them.

"Our data has been published in *NEJM* and *Muscle and Nerve* and other peer-reviewed [medical journals](#) but what people care about is more time," Frates added. "Hopefully, we can bring more time for them with their families ... and we will use that time to work hard to find a cure."

Earlier this month, an FDA advisory committee—the Peripheral and Central Nervous System Drugs Advisory Committee—voted 7–2 that the available evidence of effectiveness is sufficient to support approval of AMX0035 for the treatment of ALS.

The FDA is expected to make a decision on AMX0035 by Thursday.

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