HEALTH

New ALS drug funded by Ice Bucket Challenge already in use in South Florida trials

By Cindy Krischer Goodman
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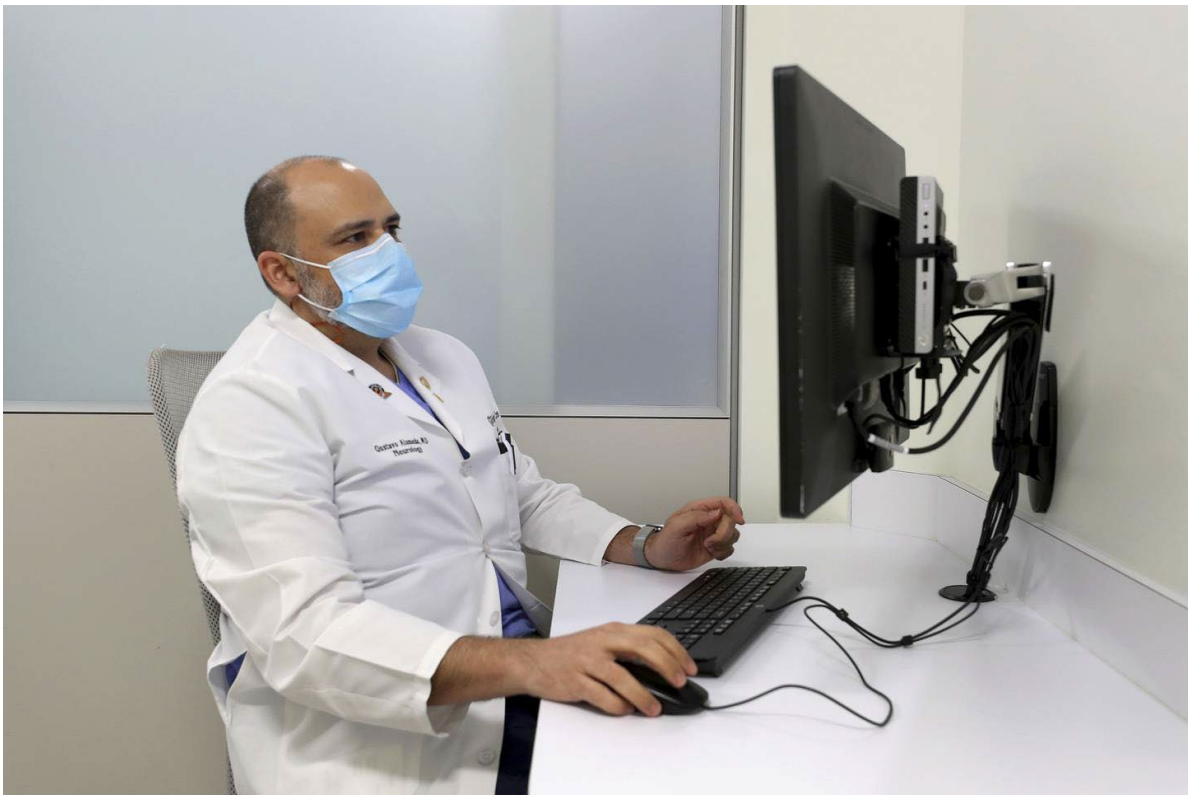
While the country awaits federal approval of a new ALS drug, people in South Florida with the disease already are using it.

The experimental drug called AMX0035 will be considered by the U.S. Food and Drug Administration on Sept. 29. and, if approved, would give patients with the degenerative disease hope for slowing the progression.

At Holy Cross Health and NSU Health Neuroscience Institute, ALS specialists began enrolling participants in a major clinical trial for the drug for amyotrophic lateral sclerosis in July. The medication is free for those in the trial, and doctors monitor patient responses.

Amylyx Pharmaceuticals, the company that makes AMX0035, asked the FDA to consider approving the drug before its larger clinical trial, the one in which Floridians are participating, is completed in early 2024. Last week, an FDA advisory panel recommended the agency approve the drug after expressing concerns earlier this year that there was not enough proof it was effective. The company is relying on data from just a single U.S. clinical trial in its application for approval.



Dr. Gustavo Alameda is heading up a clinical trial for a new ALS drug at Holy Cross Health Phil Smith Neuroscience Institute. The experimental ALS drug was funded by proceeds from the Ice Bucket Challenge, and is likely to gain FDA approval by the end of the month. People in South Florida already have access to it through the clinical trials, and local doctors participating in the trials are encouraged that it could be a good tool to slow the progression of the deadly disease with few treatment options. (Mike Stocker / South Florida Sun Sentinel)

“This is a condition that progresses and while this medication doesn’t stop it, it does slow it down,” said Dr. Eduardo Locatelli, medical director of the NSU Health Neuroscience Institute. ”We don’t have anything to cure ALS, so our patients are willing to take a risk on this.”

Locatelli said it is difficult to measure how much the drug is slowing ALS because each person’s disease progression happens at a different pace.

“We can’t compare how fast they would progress without it,” he said. He tells his patients “We think it works. if you wait two years for the data it might be too late. Being in the trial is a way to access the drug early and take a chance.“

[RELATED: Family of Parkland shooting victim sets up ALS research fund]

ALS, also known as Lou Gehrig's disease, often starts in the hands or feet and then spreads to other parts of the body and causes loss of muscle control until it becomes fatal. About 30,000 people in the United States and about 1,400 in Florida live with ALS, with about 5,000 to 6,000 people diagnosed each year.

Patients and advocacy groups in Florida are enthusiastic about the drug's potential, including the ALS Association, which funded a portion of Amylyx's study using funds from the 2014 viral Ice Bucket Challenge.

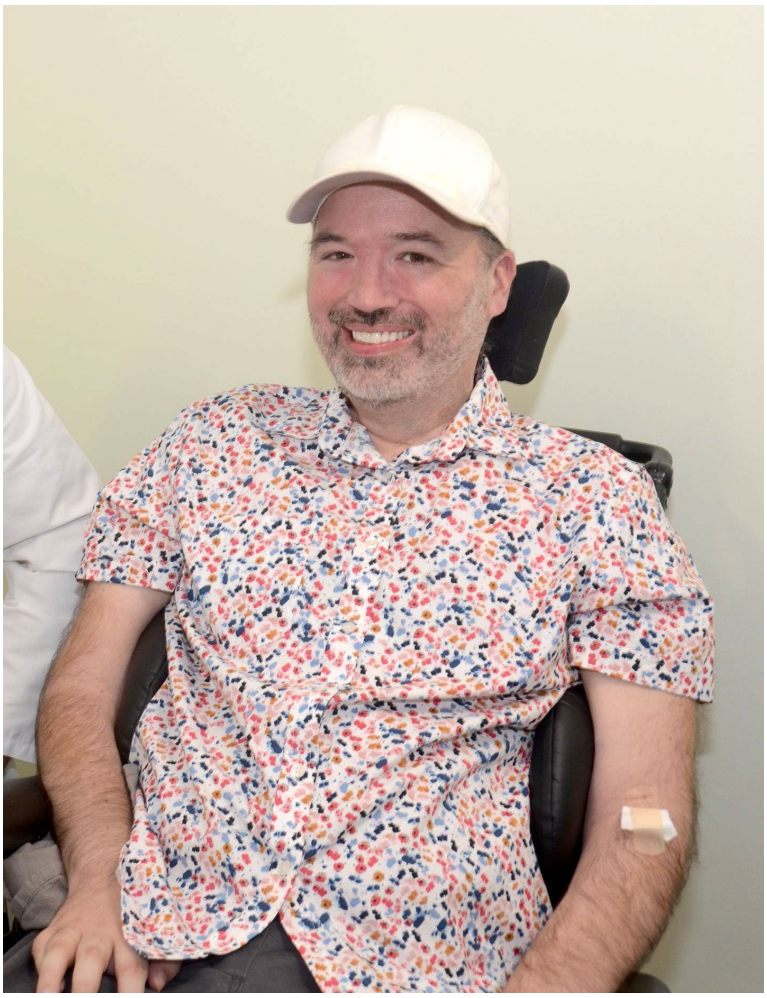
"The upside is quite significant," said Ray Carson, president and CEO of The ALS Association Florida Chapter. The prognosis for people who are diagnosed is two to five years as the disease weakens their muscles. "Even if someone gets 10 more months, that is significant. It could be the difference between being there for their daughter or son's wedding, or not."

[OPINION: Congress can give those with ALS a fighting chance]

Amylyx's AMX0035 actually is a combination of two drugs already available. One is sodium phenylbutyrate, which is prescribed and FDA-approved to treat a metabolic defect. Doctors are allowed to prescribe it off-label for ALS. The other is taurursodiol, also known as TUDCA, often sold in health food stores to help "detoxify" the liver.

The demand for both has increased with some people using compound pharmacies to create their own version of AMX0035.

Allen Walker, 44, of Coconut Creek, a father of seven, learned he had ALS 10 years ago, a rare instance of slow progression. Walker's speech is slow. He can no longer walk, and he has difficulty eating. A year ago, he began using the two-drug combination from a compound pharmacy paying as much as $500 a month. Now, he has just officially enrolled in the AMX0035 trial at Holy Cross Health. So far, Walker has seen a benefit.



Allen Walker, 44, of Coconut Creek, a patient with ALS, is enrolled in the clinical trial at Holy Cross Health for a new drug that could gain FDA approval this month. (Holy Cross/Courtesy)

“With ALS you are supposed to get worse every day, and he has been stable for the last year,” said his wife Eva. “We haven’t seen any change. He looks great and gained weight and if he stays on a steady path we are happy.”

“We believe there is hope, where in the past there hasn’t been much hope,” she said.

Dr. Gustavo Alameda, who is heading up the trial at Holy Cross Health in Fort Lauderdale, said patients who are not in the trial are using the compounded drugs. Compounding pharmacies are seeing shortages, resulting in cost increases.

“The cost has shot up for patients buying this with our prescription, and insurance coverage is spotty. A big part of FDA approval is to get more people the drug and not have a huge financial burden on them,” Alameda said.

Troy Fields, 58, a Tampa resident who was diagnosed with ALS in June 2018, has been advocating for FDA-approval of AMX0035. He says approval would make the medication more easily accessible and more likely to be covered by insurance. “In the best-case scenario, patients would get a 50% increase in their life span, which is significantly better than the current treatments,” he said.

[RELATED: Drug may extend ALS patients’ lives by several months, study finds]

Florida has six centers participating in Amylyx’s national trial: two in Broward and one each in Miami, Tampa, Gainesville and Jacksonville.

Alameda at Holy Cross said he is trying to get as many patients with ALS into the drug trial as possible. He informs them the drug won’t preserve their muscle strength or breathing but it could slow progression, according to the data already reported.

“We are kind of in a dark age for ALS, but we are coming out of it,” he said, noting that there are three additional new treatments in trial stages. There also has been some early evidence that a high dose of B12 can be beneficial for ALS patients.

Alameda said some patients do experience gastrointestinal problems from AMX0035, but most can tolerate the medication. “The disease has led us to desperation in treatment because there is so little available,” he said. “Most people will experience some reaction but they are willing to soldier through.”

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