Expert discusses new ALS drug

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The Food and Drug Administration (FDA) recently approved the first new medication for ALS (amyotrophic lateral sclerosis) in five years—despite uncertainty about how much it helps patients with the progressive and devastating neuromuscular disease.

The medication, from Amylyx Pharmaceuticals, will be sold under the name Relyvrio; it was approved based on a small Phase II clinical trial showing it slowed disease progression. Still, some medical experts have questioned the medication's effectiveness, and it took a few attempts to get the FDA green light.

Because of the severe effects of ALS—including paralysis—and the lack of solid treatments for it, the ALS community and lawmakers lobbied the FDA to be more lenient about new drugs for the condition.

Also, the medication was approved with a caveat, points out Babar Khokhar, MD, a Yale Medicine neurologist who specializes in ALS treatment.

"The FDA has to look at what the ongoing Phase III trial will show, and they won't have that data until 2024. If it reveals that the drug is not beneficial, then the company has agreed to pull it from the market," Dr. Khokhar says. "Most drugs go through the usual process of clinical trial Phases I, II, and III; if there's good efficacy and safety data, then they are approved. But ALS is so rare, and there is no treatment to stop the disease or significantly reverse it, so the FDA said they felt compelled to approve it."

We talked more with Dr. Khokhar about this medication and what it means for ALS patients.

What is ALS?

Often called Lou Gehrig's disease (which was named after the baseball player who died of it in 1941, just three years after his diagnosis), ALS is a type of motor neuron disease. Motor neurons are nerve cells that trigger all body movement, including talking, walking, breathing, and eating. ALS attacks the motor neurons, causing them to deteriorate and

eventually die.

When the nerves no longer trigger muscles, the muscles become weak, which inevitably leads to paralysis. Most people die within two to five years of an ALS diagnosis.

There is no cure for ALS, and its cause is unknown. The disease is most common in people over age 60, and men are more likely to have it than women. It is rare and there isn't an exact figure for the number of cases in the United States, but in 2017, about 31,000 Americans were estimated to have ALS.

What are the existing treatments for ALS?

There are only two approved medications to treat the symptoms of ALS in the U.S. One pill, called Rilutek, came on the market in 1995 and has been shown to extend life expectancy by two to three months. Another pill, sold as Radicava, was approved in 2017 and may slow down the disease's progression.

What do we know about this new drug, Relyvrio?

The new medication for ALS combines taurursodiol (a supplement that can regulate liver enzymes) and sodium phenylbutyrate (a medication for pediatric urea disorders, in which too much ammonia builds up in the body). It comes in a powder that can be mixed with water or administered via a feeding tube.

The medication aims to prevent motor neurons from dying. The most common reported side effects are diarrhea, abdominal pain, nausea, and upper respiratory tract infection—none of which the FDA considered significant safety risks.

It can also be taken with existing ALS medications.

What data did the FDA use to approve the medication?

The data submitted to the FDA comes from a Phase II study. The FDA typically requires favorable results from a Phase III trial before they grant approval. However, for serious diseases with few treatments, they will accept one trial, plus additional data.

In the Phase II trial, 137 patients took either Relyvrio or a placebo. Over 24 weeks, those who took the medication performed 2.32 points better on a 48-point ALS scale (which rates ALS symptoms) than those who took the placebo. That translates to about a 25% slower decline for patients receiving the treatment.

In an open-label extension study (in which patients who participated in the clinical trial were invited to keep taking the drug and be studied), 90 patients took the medication for seven more months and had a median of 4.8 months more time before being hospitalized, put on a ventilator, or dying, Amylyx reported.

Why didn't the FDA approve the medication earlier?

In March, an advisory panel to the FDA voted 6-4 against the medication, saying the data did not establish a conclusion that Relyvrio was effective in the treatment of patients with ALS. In early September, FDA advisors met again and reviewed additional evidence from the original data, plus data from the open-label extension study. This time, advisors voted 7-2 in support of approval. On Sept. 29, the FDA fully approved the medication.

What additional studies are being done?

A Phase III trial of Relyvrio is ongoing, and data is expected in 2024. As mentioned above, if the results are negative, Amylyx has said it will voluntarily remove the drug from the market.

"This drug and the other two that are available might improve the early phase of the disease, and allow patients to function and communicate more independently for longer," says Dr. Khokhar. "We usually say that if you are going to start any ALS drug, start it early. This may allow you to change the trajectory of the disease and extend the overall quality of life."

Provided by Yale University

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