New ALS drug approved in Canada while still under FDA review

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An experimental drug for the neurological disorder ALS was approved

in Canada on Monday, but an ongoing evaluation of the treatment by the U.S. Food and Drug Administration has raised questions about its effectiveness.

A condition of Health Canada's approval of Albrioza (AMX0035) calls for Massachusetts-based drug maker Amylyx Pharmaceuticals later to provide better evidence that the treatment is effective. That includes verifying the "clinical benefit of this drug" with data from an ongoing phase 3 clinical trial expected to conclude in 2024, additional pharmacological studies and periodic safety reports.

"For nearly a decade, we have been committed to creating more meaningful moments for people living with ALS and their families. We are excited with Health Canada's decision to approve Albrioza with conditions. Albrioza is a therapy that demonstrated ... a statistically significant and clinically meaningful impact on function, alone or in addition to existing ALS therapies," Justin Klee and Joshua Cohen, co-CEOs and co-founders of Amylyx, said in a company statement.

ALS (amyotrophic lateral sclerosis)—also known as Lou Gehrig's disease—often causes death within two to five years after diagnosis. There are only two approved ALS medications in the United States: riluzole, which can extend survival by several months, and edaravone, which can slow disease progression by about 33%, the *New York Times* reported.

Earlier this year, an FDA review of the drug said it was safe, but there was insufficient evidence that it helped patients live longer or slowed their loss of crucial functions such as muscle control, speaking or breathing without assistance, the *Times* reported.

In a close vote in March, an independent panel of advisers to the FDA concluded that Albrioza was not ready for approval by the agency. The

FDA recently extended its deadline for a final decision to Sept. 29, to review additional data from Amylyx.

There is a desperate need for effective ALS therapies, but when it comes to Albrioza, "it's unfortunate, but the magnitude of unmet need is not matched by the quality of evidence to date," Dr. G. Caleb Alexander, a member of the FDA's independent advisory panel, told the *Times*.

The "approval in Canada could only further increase the pressure that the FDA faces to rule favorably and to approve this product," but the FDA should still wait for the phase 3 trial results, said Alexander, an internist and epidemiologist at the Johns Hopkins Bloomberg School of Public Health in Baltimore.

Last month, 38 U.S. doctors who treat ALS patients sent a letter to the FDA urging it to approve the drug. In recent weeks, a campaign for approval of the drug has generated more than 6,000 emails asking the FDA to approve the drug, according to the ALS Association.

Amylyx bankrolled most of its research on Albrioza, but the ALS Association contributed $2.2 million raised through the 2014 Ice Bucket Challenge.

"We expect that Americans living with ALS will try to access Albrioza in Canada, just as we have heard reports of people trying to buy the ingredients on Amazon," Calaneet Balas, president and CEO of the ALS Association, told the *Times*.

Dr. Angela Genge, director of the ALS Global Centre for Excellence at the Montreal Neurological Institute, who has received fees from Amylyx for serving on an advisory board, said American patients would be legally able to receive Albrioza in Canada if it were prescribed by a Canadian physician and obtained from a Canadian pharmacy. However,

they would not be eligible for insurance coverage under Canada's public or private system.

Amylyx has not yet disclosed a price for Albrioza, the *Times* reported.

More information: Visit the U.S. National Institute of Neurological Disorders and Stroke for more on ALS.