Frequently Asked Questions about AMX0035

On June 13, 2022, Amylyx Pharmaceuticals announced that AMX0035 (known as ALBRIOZA in Canada), was approved by Health Canada. The Food and Drug Administration (FDA) has not yet approved AMX0035. The deadline for the FDA to make a decision was recently extended until September 29, 2022.

Can Americans access AMX0035 from Canada?
The legal situation is unclear. The following information is from the FDA website.

In most circumstances, it is illegal for individuals to import drugs into the United States for personal use. This is because drugs from other countries that are available for purchase by individuals often have not been approved by FDA for use and sale in the United States. For example, if a drug is approved by Health Canada (FDA's counterpart in Canada) but has not been approved by FDA, it is an unapproved drug in the United States and, therefore, illegal to import. FDA cannot ensure the safety and effectiveness of drugs that it has not approved.

FDA, however, has a policy explaining that it typically does not object to personal imports of drugs that FDA has not approved under certain circumstances, including the following situation:

- The drug is for use for a serious condition for which effective treatment is not available in the United States;
- There is no commercialization or promotion of the drug to U.S. residents;
- The drug is considered not to represent an unreasonable risk;
- The individual importing the drug verifies in writing that it is for his or her own use, and provides contact information for the doctor providing treatment or shows the product is for the continuation of treatment begun in a foreign country; and
- Generally, not more than a 3-month supply of the drug is imported.

Can Americans purchase the ingredients for AMX0035 online?
We strongly encourage people with ALS to consult with an ALS specialist, which can be found at clinics listed here: https://www.als.org/local-support/certified-centers-clinics. While it is possible to obtain the ingredients, sodium phenylbutyrate (PB) with a prescription, and taurursodiol (TURSO or TUDCA) online as a supplement, it is not possible to ensure that results would be the same as obtaining the clinically validated formulation of AMX0035 from the manufacturer.

Will Canada's decision to approve AMX0035 influence FDA?
Canada's and the United States' regulatory bodies are independent. However, Canada's decision does underscore the potential for this drug to help people with ALS. The Association has been urging the FDA to make AMX00035 available for prescribing to every American with ALS as soon as possible.

Is there anything else we can do to encourage FDA to approve AMX0035?
Yes, we have an active letter-writing campaign to the FDA at: als.org/FDA

How much will AMX0035 cost?
Amylyx has not provided cost information yet.

Will AMX0035 be covered by health insurance?
If approved by the FDA, we expect that it will be covered by health insurers. The Association has already begun to engage with health plans and ICER to ensure all ALS drugs are covered.

How long will it take for FDA to approve AMX0035?
The FDA has announced it will make a decision by September 29, 2022.

Will Amylyx be offering expanded access to AMX0035?
Amylyx is offering limited expanded access and select clinical sites. See https://www.amylyx.com/media/amylyx-pharmaceuticals-announces-launch-of-us-expanded-access-program-for-amx0035 for more information .

What were the results of AMX0035 on participants in the clinical trial?
Clinical trial participants who received AMX0035 experienced a clinically 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 act 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 functional decline.

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

How was AMX0035 funded by the ALS Association?
The ALS Association supported AMX0035 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.

Does the ALS Association receive royalty payments if AMX0035 is approved?
Our grant included a standard repayment clause of up to 150% based on the commercial sale of the drug, if and when it is approved. We will reinvest those payments in new research grants. For a listing of current research projects, see it here.

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