Important Safety and Efficacy Information on ALBRIOZA™ (sodium phenylbutyrate and ursodoxicoltaurine) – Market Withdrawal and Continued Restricted Access

2024/06/07

Audience
Healthcare professionals including neurologists and other healthcare professionals engaged in the care of patients with amyotrophic lateral sclerosis (ALS).

Key messages

- **Amylyx Pharmaceuticals, Inc. will withdraw ALBRIOZA from the Canadian market by December 31, 2024.**
- **In 2022, ALBRIOZA (sodium phenylbutyrate and ursodoxicoltaurine) was authorized under the Notice of Compliance with Conditions (NOC/c) policy for the treatment of adult patients with amyotrophic lateral sclerosis (ALS).**
- **The Phase 3 confirmatory study did not meet its primary or secondary endpoints.**
- **Healthcare professionals are advised to:**
  - **NOT initiate ALBRIOZA in new patients. ALBRIOZA is now available only under Amylyx Pharmaceuticals, Inc.'s Patient Support Program for patients currently receiving treatment with ALBRIOZA.**
  - **Discuss with their patients whether to continue treatment with ALBRIOZA or switch to an alternative drug.**
- **The Canadian Product Monograph (CPM) for ALBRIOZA has been updated to reflect this new information. Health Canada will continue to work with the manufacturer throughout the market withdrawal process.**

What is the issue?
In 2022, ALBRIOZA was authorized under the NOC/c policy for the treatment of patients with ALS, based on results from a Phase 2 clinical study and pending the results of trials to verify its clinical benefit.

ALBRIOZA is now available only to current patients under Amylyx Pharmaceuticals, Inc.'s Patient Support Program and should not be initiated in new patients. This is due to the results of a Phase 3 confirmatory study that did not meet its primary or

secondary endpoints and led to the decision to withdraw the product from the market.

Products affected
ALBRIOZA; 3 g of sodium phenylbutyrate and 1 g of ursodoxicoltaurine per sachet; powder for oral suspension.

Background information
ALBRIOZA is indicated for the treatment of adult patients with ALS.

In 2022, ALBRIOZA was authorized under the NOC/c policy based on results from a Phase 2, multi-centre, randomized, double-blind, placebo-controlled, parallel-group clinical study in 137 participants with familial or sporadic ALS.

Authorization under the NOC/c policy was contingent on verification of clinical benefit in a Phase 3, multi-centre, randomized study comparing efficacy and safety of ALBRIOZA versus placebo. The confirmatory study was not submitted to Health Canada for further review as it did not meet its primary efficacy endpoint. There was no significant difference observed between participants treated with ALBRIOZA and placebo in the Revised Amyotrophic Lateral Sclerosis Functional Rating Scale (ALSFRS-R) total score change from baseline. There were also no statistically significant improvements observed in the secondary efficacy endpoints.

At this time, the clinical benefit of ALBRIOZA for the treatment of patients with ALS remains unconfirmed.

Amylyx Pharmaceuticals, Inc. will withdraw ALBRIOZA from the Canadian market by December 31, 2024. New patients should not be initiated on ALBRIOZA.

Information for consumers
ALBRIOZA is a prescription medicine used to treat people with ALS.

ALBRIOZA was authorized with conditions under the NOC/c policy based on promising evidence of clinical efficacy following Health Canada's review. The manufacturer agreed to complete more studies to ensure that the drug works as expected.

In a recent Phase 3 study, ALS participants received either ALBRIOZA or placebo. This study failed to show that ALBRIOZA was more effective than placebo in slowing the progression of ALS.

Patients should discuss any questions or concerns about this information with their healthcare professional. Patients should continue to inform their healthcare professional if they are experiencing any side effects while receiving ALBRIOZA.

Information for healthcare professionals
Amylyx Pharmaceuticals, Inc. will withdraw ALBRIOZA from the Canadian market by December 31, 2024.

Healthcare professionals are advised of the following:

- ALBRIOZA should not be initiated in new patients.
- ALBRIOZA is now only available under Amylyx Pharmaceuticals, Inc.'s Patient Support Program for patients who wish to continue receiving ALBRIOZA.
- For more information on the Patient Support Program, contact Amylyx Pharmaceuticals, Inc.'s patient support program at 1-877-710-0711 or support@amylyxcareteam.ca.

Action taken by Health Canada

Health Canada has worked with the manufacturer to update the CPM for ALBRIOZA to include this new information. Health Canada will continue to work with the manufacturer throughout the market withdrawal process.

Health Canada is communicating this important information to healthcare professionals and Canadians via the Recalls and Safety Alerts Database on the Healthy Canadians Web Site. This communication will be further distributed through the MedEffect™ e-Notice email notification system.

Report health or safety concerns

Managing marketed health product-related side effects depends on healthcare professionals and consumers reporting them. Any case of serious or unexpected side effects in patients receiving ALBRIOZA should be reported to Amylyx Pharmaceuticals, Inc. or Health Canada.

Amylyx Pharmaceuticals, Inc.
43 Thorndike Street
Cambridge, MA
02141
Tel: 1-877-710-0711

To correct your mailing address or fax number, contact Amylyx Pharmaceuticals, Inc.

You can report any suspected adverse reactions associated with the use of health products to Health Canada by:
- Calling toll-free at 1-866-234-2345; or
- Visiting MedEffect Canada's Web page on Adverse Reaction Reporting for information on how to report online, by mail or by fax.

For other health product inquiries related to this communication, contact Health Canada at:
Pharmaceutical Drugs Directorate
E-mail: pharma_drug_enquiries-renseignements_medicaments_pharma@hc-sc.gc.ca
Telephone: 613-957-0368
Fax: 613-952-7756

Original signed by

S. Machelle Manuel, Ph.D.
Global Head of Medical Affairs
Amylyx Pharmaceuticals, Inc.