CNS Drugs

PEER-REVIEWED FEATURE

Sodium Phenylbutyrate and Ursodoxicoltaurine: Adis Evaluation

Key points

- An oral fixed-dose coformulation being developed by Amylyx Pharmaceuticals for the treatment of neurodegenerative diseases
- Received its first approval with conditions on 13 June 2022 in Canada
- Approved for the treatment of amyotrophic lateral sclerosis (ALS) in adults

Summary

An oral, fixed-dose coformulation of sodium phenylbutyrate and ursodoxicoltaurine (ALBRIOZA™; hereafter denoted sodium phenylbutyrate/ursodoxicoltaurine) is being developed by Amylyx Pharmaceuticals for the treatment of ALS and other neurodegenerative diseases.

The coformulation is designed to reduce neuronal cell death by attenuating endoplasmic reticulum stress and mitochondrial dysfunction.

On 13 June 2022, sodium phenylbutyrate/ursodoxicoltaurine received its first approval with conditions in Canada for the treatment of ALS in adults.

Sodium phenylbutyrate/ursodoxicoltaurine is undergoing regulatory review in the USA and EU for the treatment of ALS. In the USA, the coformulation is also undergoing phase II clinical development for Alzheimer's disease and preclinical development for Wolfram syndrome.