



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Refusal of the marketing authorisation for Albriozza (*sodium phenylbutyrate / ursodoxicoltaurine*)

Re-examination confirms refusal

After re-examining its initial opinion, the European Medicines Agency has confirmed its recommendation to refuse marketing authorisation for the medicine Albriozza. The medicine was intended for the treatment of amyotrophic lateral sclerosis.

The Agency issued its opinion after re-examination on 12 October 2023. The Agency had issued its initial opinion on 22 June 2023. The company that applied for authorisation of Albriozza is Amylyx Pharmaceuticals EMEA B.V.

What is Albriozza and what was it intended for?

Albriozza was developed as a medicine for adults with amyotrophic lateral sclerosis (ALS). ALS is a progressive disease of the nervous system where nerve cells in the brain and spinal cord that control voluntary movement gradually deteriorate, causing loss of muscle function and paralysis. The medicine contains two active substances, sodium phenylbutyrate and ursodoxicoltaurine, and was to be available as a powder to be dissolved in water and taken by mouth.

Albriozza was designated an 'orphan medicine' (a medicine used in rare diseases) on 4 June 2020 for the treatment of ALS. Further information on the orphan designation can be found on the Agency's website: ema.europa.eu/medicines/human/orphan-designations/eu-3-20-2284.

How does Albriozza work?

It is not fully clear how Albriozza works, but the two active substances, sodium phenylbutyrate and ursodoxicoltaurine, were expected to reduce damage to nerve cells and prevent them from dying. This was expected to help maintain normal muscle function and slow down the worsening of the disease.

What did the company present to support its application?

The company provided results from one main study involving 137 patients with ALS who received either Albriozza or placebo (a dummy treatment) in addition to their standard treatment over 24 weeks. The main measure of effectiveness was the rate at which patients' symptoms, such as difficulty talking,

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breathing, eating and performing other normal daily activities, worsened during the study. This was assessed using a standard rating scale known as 'ALS functional rating scale revised' (ALSFRS-R). The company also provided results on the overall survival time.

What were the main reasons for refusing the marketing authorisation?

At the time of the initial opinion in June 2023, the Agency had concerns that the main study did not show convincingly that Albriozza was effective in slowing down the worsening of the disease. Data on survival were also not reliable, given the way the data were collected and analysed. Therefore, the Agency's opinion was that a positive balance of benefits and risks of Albriozza could not be established. Hence, the Agency recommended refusing marketing authorisation. In giving this recommendation, the Agency also considered advice from expert groups, including patient representatives and experts in neurology.

During the re-examination, the Agency assessed the company's responses to its concerns and consulted a group of experts in neurology. After re-examination, the Agency's concerns were not resolved and the initial refusal was therefore confirmed.

The Agency also took into consideration several third party interventions from ALS patient associations, ALS advocates and a national neurology association.

Does this refusal affect patients in clinical trials?

The company informed the Agency that there are no consequences for patients currently included in clinical trials with Albriozza. If you are in a clinical trial and need more information about your treatment, speak with your clinical trial doctor.