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EPAR summary for the public

Brineura

cerliponase alfa

This is a summary of the European public assessment report (EPAR) for Brineura. It explains how the Agency assessed the medicine to recommend its authorisation in the EU and its conditions of use. It is not intended to provide practical advice on how to use Brineura.

For practical information about using Brineura, patients should read the package leaflet or contact their doctor or pharmacist.

What is Brineura and what is it used for?

Brineura is a medicine for treating neuronal ceroid lipofuscinosis type 2 (CLN2 disease), an inherited condition in children that leads to progressive brain damage.

Because the number of patients with CLN2 is low, the disease is considered 'rare', and Brineura was designated an 'orphan medicine' (a medicine used in rare diseases) on 13 March 2013.

Brineura contains the active substance cerliponase alfa.

How is Brineura used?

Brineura is infused directly into the brain. Before the first infusion, the patient will need to have surgery to implant a device, which runs from the outside of the skull through to the fluid cavity in the brain where the medicine is delivered to.

The infusions are given once every two weeks by a healthcare professional knowledgeable about giving medicines into the brain. To reduce the risk of infusion-related reactions, patients may be given other medicines before or during treatment with Brineura or the infusion may be slowed down. The treatment can continue for as long as the patient benefits from it.

Brineura can only be obtained with a prescription. For further information, see the package leaflet.

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How does Brineura work?

Patients with CLN2 lack an enzyme required for normal brain development called tripeptidyl-peptidase-1 (TPP1). The active substance in Brineura, cerliponase alfa, is a copy of TPP1 and is used as a replacement for the missing enzyme.

The medicine is infused directly into the brain in order to bypass the blood-brain barrier, a protective barrier that separates the blood stream from the brain, and prevents substances such as medicines, from entering brain tissue.

What benefits of Brineura have been shown in studies?

Brineura has been shown in early studies to reduce the rate at which the disease worsens as measured with standard rating scale.

In a study in which 23 children (average age of 4 years) were treated with Brineura for almost a year, 20 of them (87%) did not experience the 2-point decline in movement and language skills seen historically in patients not receiving treatment.

The rating was done by doctors who gave patients individual scores for movement and language skills (where 0 means most severe and 3 is normal). A patient's final score was the sum of the two scores.

In a follow-up study the benefits of Brineura lasted for another year, with the results showing the disease could be delayed in the majority of patients. This study is still ongoing.

What are the risks associated with Brineura?

The most common side effects with Brineura (which may affect more than 1 in 5 people) are fever, low levels of protein in CSF (the fluid in the brain and spinal cord), abnormalities in ECG (a test of the heart's activity), vomiting, upper respiratory tract infections (nose and throat infections), and hypersensitivity (allergic) reactions. For the full list of all side effects reported with Brineura, see the package leaflet.

Brineura must not be given to patients who have experienced life-threatening hypersensitivity (allergic) reactions with Brineura and whose symptoms re-occurred when Brineura was given again. It must also not be given to patients who have had a shunt implanted to drain extra fluid from the brain. Finally, patients must not be given Brineura while there are any problems with the device, such as leakage or infection.

Why is Brineura approved?

Available data show that Brineura helps slow the decline in movement and language skills in patients with CLN2, a condition for which there are no other treatments.

With regard to its safety, the data do not reveal any unacceptable concerns. The Agency's Committee for Medicinal Products for Human Use (CHMP) therefore concluded that Brineura's benefits are greater than its risks and recommended that it be approved for use in the EU.

Brineura has been authorised under 'exceptional circumstances'. This is because it has not been possible to obtain complete information about Brineura due to the rarity of the disease. Every year, the European Medicines Agency will review any new information that becomes available and this summary will be updated as necessary.

What information is still awaited for Brineura?

Since Brineura has been approved under exceptional circumstances, the company that markets it will provide further data from studies on the safety of Brineura, including the risk of allergic reactions when used long-term, and on its long-term effectiveness in delaying or stopping worsening of movement and language skills. The studies will include children below 2 years of age, for whom there are currently no data.

What measures are being taken to ensure the safe and effective use of Brineura?

The company that markets Brineura will ensure that all healthcare professionals expected to use this medicine are provided with training material on how to use it and how to avoid problems with the device such as infection or blockage.

Recommendations and precautions to be followed by healthcare professionals and patients for the safe and effective use of Brineura have also been included in the summary of product characteristics and the package leaflet.

Other information about Brineura

The European Commission granted a marketing authorisation valid throughout the European Union for Brineura on 30 May 2017.

The full EPAR for Brineura can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/European public assessment reports. For more information about treatment with Brineura, read the package leaflet (also part of the EPAR) or contact your doctor or pharmacist.

The summary of the opinion of the Committee for Orphan Medicinal Products for Brineura can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/Rare disease designation.

This summary was last updated in 05-2017.