Package leaflet: Information for the patient

Brineura 150 mg solution for infusion
cerliponase alfa

▼This medicine is subject to additional monitoring. This will allow quick identification of new safety information. You can help by reporting any side effects you may get. See the end of section 4 for how to report side effects.

Read all of this leaflet carefully before you or your child is given this medicine because it contains important information for you.
- Keep this leaflet. You may need to read it again.
- If you have any further questions, ask your doctor.
- If you or your child get any side effects, talk to your doctor.
- This includes any possible side effects not listed in this leaflet. See section 4.

What is in this leaflet

1. What Brineura is and what it is used for

Brineura contains the active substance cerliponase alfa, which belongs to a group of medicines known as enzyme replacement therapies. It is used to treat patients with neuronal ceroid lipofuscinosis type 2 (CLN2) disease, also known as tripeptidyl peptidase-1 (TPP1) deficiency.

People with CLN2 disease do not have any enzyme called TPP1 or they have too little of it and this causes a build-up of substances called lysosomal storage materials. In people with CLN2 disease, these materials build-up in certain parts of the body, mainly the brain.

How Brineura works
This medicine replaces the missing enzyme, TPP1, which minimises the build-up of the lysosomal storage materials. This medicine works to slow the progression of the disease.

2. What you need to know before you or your child is given Brineura

You must not receive Brineura
- If you or your child has had life-threatening allergic reactions to cerliponase alfa or any of the other ingredients of this medicine (listed in section 6), and the reactions continue to happen when cerliponase alfa is given again.
- If you or your child has a device implanted to drain extra fluid from the brain.
- If you or your child currently has signs of a device infection or problems with the device. Your doctor may decide to continue treatment once the device infection or problems are resolved.

Warnings and precautions
Talk to your doctor before you or your child is given Brineura.
- You or your child may get problems with the implanted device used during treatment with Brineura (see section 4 “Possible side effects”), including infection or a fault in the device. Signs that you or your child may have an infection include fever, headache, neck stiffness, light sensitivity, nausea, vomiting, and change in mental status. Signs of problems with the device include swelling, redness of the scalp, fluid leaking from device and bulging of the scalp. Treatment may be interrupted if the device needs to be replaced or until the infection clears. Within 4 years of use, the access device may

need to be replaced and will be determined by your doctor. Talk to your doctor if you have any questions about your device.
- Life-threatening allergic reactions (anaphylactic reactions) are possible with this medicine. Your doctor will monitor you or your child for symptoms of life threatening allergic reactions, such as hives, itching or flushing, swollen lips, tongue, and/or throat, chills, accelerated heart rhythm, shortness of breath, hoarseness, turning blue around finger tips or lips, low muscle tone, fainting, diarrhoea or incontinence. Seek immediate medical care should these symptoms occur.
- Your doctor will check your or your child's heart rate, blood pressure, respiratory rate, and temperature before, during, and after treatment. The doctor may decide on additional monitoring if it is needed.
- Your doctor will check for abnormal heart electrical activities (ECG) every 6 months. If you or your child has a history of heart problems, your doctor or nurse will monitor your heart activity during each infusion.
- Your doctor may send samples of brain fluid to check for signs of infection.
- This medicine has not been given to patients with advanced disease at the start of treatment or in children younger than 1 year of age. Your doctor will discuss whether Brineura treatment is right for you or your child.

Other medicines and Brineura
Tell your doctor if you or your child is taking, has recently taken, or might take any other medicines.

Pregnancy, breast-feeding and fertility
If you are pregnant or breast-feeding, think you may be pregnant, or are planning to have a baby, ask your doctor for advice before treatment with this medicine.

You should not receive this medicine during pregnancy unless clearly necessary. It is not known if this medicine can harm your unborn baby.

You should not receive this medicine if you are breast-feeding. It is not known if this medicine passes into human breast milk.

It is not known if Brineura impacts human fertility.

Driving and using machines
It is not known if this medicine will impact the ability to drive or use machines. Please consult your doctor.

Brineura contains sodium and potassium
This medicine contains 17.4 mg sodium (main component of cooking/table salt) in each vial. This is equivalent to 0.87% of the recommended maximum daily dietary intake of sodium for an adult.

This medicine contains potassium, less than 1 mmol (39 mg) per vial, that is to say essentially 'potassium-free'.

3. How Brineura is given

You or your child will need to have surgery to implant the device for givingthis medicine. The device helps the medicine to reach a specific part of the brain.

This medicine will be given by a doctor with knowledge of giving medicines by intracerebroventricular use (infusion into the fluid of the brain) in a hospital or clinic.

Brineura has not been given to patients younger than 1 year of age or older than 9 years of age (at the start of the clinical trial).

The recommended dose of this medicine is based upon your or your child's age, and is given once every other week as follows:

- birth to < 6 months: 100 mg
- 6 months to < 1 year: 150 mg
- 1 year to < 2 years: 200 mg (first 4 doses), 300 mg (all other doses)
- ≥ 2 years: 300 mg

Your doctor may adjust you or your child's dose or the amount of time the medicine is given if the infusion is not tolerated, there is an allergic reaction or there is a possible increase of pressure in the brain.

The medicine is slowly pumped through the implanted device. After the medicine has been given, a shorter infusion of a solution is given to flush Brineura out of the infusion equipment so that the full dose reaches the brain. The medicine and solution will be given over about 2 to 4 hours and 30 minutes according to your or your child's dose. Your doctor may lower the dose or the speed of the infusion based on your response during the treatment.

Your doctor may give you or your child medicines, such as antipyretics to reduce fever or antihistamines to treat allergic reactions before each treatment with this medicine to reduce side effects that can occur during or shortly after treatment.

If you have any further questions on the use of this medicine, ask your doctor.

4. Possible side effects

Like all medicines, this medicine can cause side effects, although not everybody gets them.

Talk to your doctor or nurse immediately if you experience any of the following:

Very common side effects (may affect more than 1 in 10 people):
- convulsions (seizures)
- reactions during or shortly after being given the medicine, such as hives, itching or flushing, swollen lips, tongue and/or throat, shortness of breath, hoarseness, turning blue around finger tips or lips, low muscle tone, fainting or incontinence.
- device-related bacterial infections

Common side effects (may affect up to 1 in 10 people):
- severe allergic reaction (anaphylactic reactions).

Not known (frequency cannot be estimated from the available data):
- inflammation of the brain (meningitis) due to device-related infection.

This medicine may cause other side effects:

Very common side effects (may affect more than 1 in 10 people):
- fever
- vomiting
- feeling irritable
- headache
- increased or decreased protein in the brain fluid detected by laboratory monitoring
- abnormal results of heart electrical activity (ECG)
- increased cells in the spinal fluid detected by laboratory monitoring
- device does not function correctly due to a blockage detected during preparation for infusion
- leakage of the device
- needle issue (infusion needle falls out of implanted device).

Common side effects (may affect up to 1 in 10 people):
- slower heart beat
- rash
- hives

- device breakage
- device site irritation
- feeling nervous
- disorder of the stomach or intestines.

Not known (frequency cannot be estimated from the available data):
- device is displaced and does not function correctly when preparing for infusion.

Reporting of side effects
If you or your child gets any side effects, talk to your doctor. This includes any possible side effects not listed in this leaflet. You can also report side effects directly (see details below). By reporting side effects, you can help provide more information on the safety of this medicine.

United Kingdom
Yellow Card Scheme
Website: www.mhra.gov.uk/yellowcard or search for MHRA Yellow Card in the Google Play or Apple App Store

5. How to store Brineura

Keep this medicine out of the sight and reach of children.

Do not use this medicine after the expiry date, which is stated on the vials and carton after EXP. The expiry date refers to the last day of that month.

Store upright in a freezer (-25°C to -15°C). Store in the original package, in order to protect from light. Transport and distribute frozen (-85°C to -15°C).

Thawed Brineura and flushing solution should be used immediately. This medicine should only be withdrawn from the unopened vials immediately prior to use. If immediate use is not possible, unopened vials of Brineura or flushing solution should be stored in a refrigerator (2°C to 8°C) and used within 24 hours.

Chemical and physical in-use stability has been demonstrated for up to 12 hours at room temperature (19°C to 25°C). From a microbiological point of view, open vials or medicinal product held in syringes should be used immediately. If not used immediately, in-use storage times and conditions prior to use are the responsibility of the user.

Your doctor or pharmacist is responsible for storing Brineura. They are also responsible for disposing of any unused Brineura properly.

6. Contents of the pack and other information

What Brineura contains
- The active substance is cerliponase alfa. Each vial of Brineura contains 150 mg of cerliponase alfa in 5 ml of solution. Each ml of solution for infusion contains 30 mg of cerliponase alfa.
- The other ingredients of Brineura solution for infusion and the flushing solution are: sodium phosphate dibasic heptahydrate, sodium dihydrogen phosphate monohydrate, sodium chloride, potassium chloride, magnesium chloride hexahydrate, calcium chloride dihydrate, and water for injections (see section 2 “Brineura contains sodium and potassium”).

What Brineura looks like and contents of the pack
Brineura and the flushing solution are solutions for infusion. The Brineura solution for infusion is clear to slightly opalescent, colourless to pale yellow that may occasionally contain thin translucent fibres or opaque particles. The flushing solution is clear and colourless.

Pack size: Each pack contains two vials of Brineura solution for infusion and one vial of flushing solution, each containing 5 ml of solution

Marketing Authorisation Holder and Manufacturer
BioMarin International Limited
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This medicine has been authorised under 'exceptional circumstances'. This means that because of the rarity of this disease it has been impossible to get complete information on this medicine.

Other sources of information

Detailed information on this medicine is available on the website of the Medicines and Healthcare products Regulatory Agency https://products.mhra.gov.uk.