Brineura®

Consumer Medicine Information (CMI) summary

The full CMI on the next page has more details. If you are worried about your child using this medicine, speak to your doctor or pharmacist.

1. Why is my child using Brineura?

Brineura contains the active ingredient cerliponase alfa. Brineura is used to treat patients with neuronal ceroid lipofuscinosis type 2 disease, also known as tripeptidyl peptidase 1 deficiency.

For more information, see Section 1. Why is my child using Brineura? in the full CMI.

2. What should I know before my child is given Brineura?

Do not use if your child has ever had an allergic reaction to Brineura or any of the ingredients listed at the end of the CMI.

Talk to your child's doctor if your child has any other medical conditions or take any other medicines.

For more information, see Section 2. What should I know before my child is given Brineura? in the full CMI.

3. What if my child is taking other medicines?

Some medicines may interfere with Brineura and affect how it works.

Tell your child's doctor or pharmacist if your child is taking any other medicines.

A list of these medicines is in Section 3. What if my child is taking other medicines? in the full CMI.

4. How does my child use Brineura?

Your child will need to have surgery to implant the device for giving Brineura. The device helps the medicine to reach a specific part of the brain. The recommended dose of Brineura is based upon your child's age and is given once every two weeks by a doctor in a hospital or clinic.

More information can be found in Section 4. How does my child use Brineura? in the full CMI.

5. What should I know while my child is receiving Brineura?

Things you should do	Keep all appointments with the doctor. Remind any doctor, dentist or pharmacist you visit that your child is using Brineura.
Things you should not do	Do not stop your child's treatment visits for Brineura unless you have spoken to your child's doctor.
Driving or using machines	After Brineura treatment, be careful before your child drives, rides a bike or uses any machines or tools until you know how Brineura affects them.

For more information, see Section 5. What should I know while my child is receiving Brineura? in the full CMI.

6. Are there any side effects?

All medicines can have side effects. If they do occur, they are usually minor and temporary. Do not be alarmed by this list. Your child may not experience any of them. Serious side effects include symptoms of an allergic reaction, signs of an infection in the implanted device, and seizures. Common side effects include stomach complaints, vomiting, fever, feeling irritable or nervous, rash or hives, pain, headache, and slower heartbeat. For more information, including what to do if your child has any side effects, see Section 6. Are there any side effects? in the full CMI.

Brineura®

Active ingredient: *cerliponase alfa* (ser LIP oh nase AL fa)

Consumer Medicine Information (CMI)

This leaflet provides important information about using Brineura. **You should also speak to your child's doctor or pharmacist if you would like further information or if you have any concerns or questions about your child using Brineura.**

Where to find information in this leaflet:

1. Why is my child using Brineura?

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

CLN2 disease is an inherited condition that causes progressive irreversible decline in ability to speak and move, loss of balance, convulsions (seizures), blindness and ultimately, death in children. Brineura has been shown to reduce the rate of decline in ability to speak and move.

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.

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

2. What should I know before my child is given Brineura?

Warnings

Do not use Brineura if:

- your child has had life-threatening allergic reactions to cerliponase alfa or any of the other ingredients of this medicine listed at the end of this leaflet, and the reactions continue to happen when cerliponase alfa is given again. Always check the ingredients at the end of this leaflet to make sure your child can use this medicine.
- your child has a device implanted to drain extra fluid from the brain.
- your child currently has signs of a device infection or problems with the device used to give Brineura. Your doctor may decide to continue treatment once the device infection or problems are resolved.

Brineura is given only by trained healthcare professionals knowledgeable in intracerebroventricular administration in a healthcare setting.

Because reactions to infusion of Brineura are common the doctor will usually give an anti-allergy medication about 30 minutes before Brineura infusion to reduce the severity of these reactions. The reactions may include fever, vomiting, and irritability. The doctor may reduce the infusion rate or dose if the reaction is severe.

Brineura has not been given to patients with advanced disease at the start of treatment or in children younger than 1 year of age. The doctor will discuss whether Brineura treatment is right for your child.

Check with your child's doctor if your child:

- is on a controlled sodium diet. Each vial of Brineura contains 17.42 mg sodium
- has any other medical conditions
- takes any medicines for any other condition

During treatment, your child may be at risk of developing certain side effects. It is important you understand these risks and how to monitor for them. See additional information under Section 6. Are there any side effects?

Before starting treatment with Brineura, talk to your child's doctor about

The implanted access device

- Because Brineura is administered into the brain, surgery to implant a reservoir and catheter (intracerebroventricular access device) must be performed first.
- Your child may get problems with the implanted device used during treatment with Brineura (see "Side effects"), including infection or a fault in the device.
  - Signs that 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.
- After long periods of use, the access device may need to be replaced and this will be determined by the doctor.

Medical checks of your child while taking Brineura

- The doctor will check 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.
- The doctor will check for abnormal heart electrical activities (ECG) every 6 months. If your child has a history of heart problems, the doctor or nurse will monitor your child's heart activity during each infusion.
- Your doctor may send samples of brain fluid to check for signs of infection.

3. What if my child is taking other medicines?

Tell your doctor or pharmacist if your child is taking any other medicines, including any medicines, vitamins or supplements that you buy without a prescription from your pharmacy, supermarket or health food shop.

Check with your child's doctor or pharmacist if you are not sure about what medicines, vitamins or supplements your child is taking and if these affect Brineura.

4. How does my child use Brineura?

How Brineura is given

Your child will need to have surgery to implant the device for giving Brineura. The device helps the medicine to reach a specific part of the brain.

Brineura 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.

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 child's dose.

How much is given

The recommended dose of Brineura is based upon your child's age and is given once every two weeks 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 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.

How long to use Brineura

The doctor will decide how long your child will receive Brineura.

Your child will be reviewed regularly to see if they continue to benefit from treatment.

If your child forgets to use Brineura

If your child misses their dose at the usual time, talk to their doctor or nurse and arrange another visit as soon as possible.

5. What should I know while my child is receiving Brineura?

Things you should do

- Keep all appointments with the doctor and always discuss anything that worries you or your child during or after treatment with Brineura.
- Before starting any new medicine, remind the doctor or pharmacist that your child is receiving Brineura.
- Tell all the doctors, dentists and pharmacists who are treating your child that they are receiving Brineura.

Things you should not do

- Do not stop your child's treatment visits for Brineura unless you have spoken to your doctor. Your child's condition may worsen if they stop receiving Brineura.

Driving or using machines

After Brineura treatment, be careful before your child drives, rides a bike or uses any machines or tools until you know how Brineura affects them.

Looking after your medicine

Brineura must be stored upright in a freezer (-25°C to -15°C). It should be stored in the carton to protect it from light.

Each vial of Brineura and flushing solution are intended for single use in one patient only. Discard any residue.

Brineura should not be given if the expiry date printed on the carton has passed.

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

6. Are there any side effects?

All medicines can have side effects. If your child does experience any side effects, most of them are minor and

temporary. However, some side effects may need medical attention.

See the information below and, if you need to, ask your child's doctor or pharmacist if you or your child have any further questions about side effects.

Less serious side effects

Less serious side effects	What to do
Gastrointestinal-related: • Vomiting • Stomach pain **General body-related:** • Fever • Feeling irritable or nervous • Pain • Headache • Rash or hives **Heart-related:** • A slower heartbeat	**Speak to your doctor if your child has any of these less serious side effects and they worry you or your child.**

Serious side effects

Serious side effects	What to do
Allergic reaction-related: • Hives, itching or flushing • Swollen lips, tongue and/or throat • Shortness of breath • Hoarseness • Turning blue around fingertips or lips • Low muscle tone • Fainting • Incontinence **Device infection-related:** • Symptoms of brain inflammation e.g. fever, headache, stiff neck, light sensitivity, nausea, vomiting, change in mental status (confusion, change in behaviour) **Nervous system-related:** • Convulsions (seizures)	**Call your doctor straight away, or go straight to the Emergency Department at your nearest hospital if you or your child notices any of these serious side effects.**

Your child may get problems with the implanted device used during treatment with Brineura, such as incorrect function due to a blockage, movement or leakage of the device, or an issue with the needle. The doctor or nurse will monitor for these problems and make changes to your child's treatment to deal with them if they occur.

Tell your child's doctor or pharmacist if you notice anything else that may be making your child feel unwell.

Other side effects not listed above may also occur in some patients, e.g. abnormal results of heart electrical activity (ECG), increased cells in the spinal fluid and increased or decreased protein in the brain fluid. These side effects can only be found when the doctor does tests from time to time to check your child's progress.

Reporting side effects

After you have received medical advice for any side effects your child experiences, you can report side effects to the Therapeutic Goods Administration online at www.tga.gov.au/reporting-problems. By reporting side effects, you can help provide more information on the safety of this medicine.

Always make sure you speak to your child's doctor before your child decides to stop taking any of their medicines.

7. Product details

This medicine is only available with a doctor's prescription.

What Brineura contains

Active ingredient (main ingredient)	150 mg cerliponase alfa in each vial
Other ingredients (inactive ingredients)	• dibasic sodium phosphate heptahydrate • monobasic sodium phosphate monohydrate • sodium chloride • potassium chloride • magnesium chloride hexahydrate • calcium chloride dihydrate • water for injections The flushing solution contains the inactive ingredients only. Brineura and flushing solution do not contain preservative.

Do not take this medicine if your child is allergic to any of these ingredients.

What Brineura looks like

Brineura solution is clear to slightly opalescent, colourless to pale yellow; the Brineura solution may occasionally contain thin semi-transparent fibres or opaque particles. The flushing solution is clear and colourless.

Both solutions are supplied in clear glass vials with a plastic flip-off cap and aluminium seal. Each pack contains 2 vials of Brineura and 1 vial of flushing solution, each containing 5 mL of solution. (Aust R 298672).

Who distributes Brineura

BioMarin Pharmaceutical Australia Pty Ltd
119 Willoughby Road
Crows Nest, NSW 2065
Telephone (02) 8520 3255

For enquiries about Brineura, contact medinfo@bmrn.com or call BioMarin on 1800 387 876.

To report adverse events, contact drugsafety@bmrn.com or call BioMarin Australia on 1 800 387 876.

This leaflet was prepared in November 2024.