▼ This medicine is subject to additional monitoring. This will allow quick identification of new safety information. You can help by reporting any side affects you may get. You can report side effects to your doctor, or directly at www.tga.gov.au/reporting-problems

BRINEURA

cerliponase alfa (rch)

Consumer Medicine Information

What is in this leaflet

This leaflet answers some common questions about Brineura.

It does not contain all the available information. It does not take the place of talking to the doctor or pharmacist.

All medicines have benefits and risks. The doctor has weighed the risks of treating your child with Brineura against the expected benefits .

If you have any concerns about this medicine, talk to the doctor, nurse or the hospital pharmacist.

Keep this leaflet while your child is being treated with Brineura.

You may need to read it again.

What Brineura 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.

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.

Brineura has not been given to patients with advanced disease at the start of treatment or in children younger than 2 years of age. The doctor will discuss whether Brineura treatment is right for your child.

Brineura is a new medicine. It has been used in a limited number of children and its long term effects are not yet known.

How Brineura works

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.

Ask the doctor if you have any questions about why Brineura has been prescribed for your child.

Brineura is available only with a doctor's prescription.

Before starting treatment with Brineura

When you must not have it

Your child must not receive Brineura:

- if your child has had life-threatening allergic reactions to cerliponase alfa or any of the other ingredients of this medicine (listed under “Ingredients”), and the reactions continue to happen when cerliponase alfa is given again.
- if your child has a device implanted to drain extra fluid from the brain.
- if 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.

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

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

Brineura should not be given if the packaging is torn or shows signs of tampering.

The nurse or hospital pharmacist will check this.

Check with the doctor, nurse or pharmacist if you are not sure about any of the above.

Before starting treatment with Brineura

Tell the doctor if your child has allergies to any other medicines, foods, preservatives or dyes.

Because reactions to infusion of Brineura are common it is usual to 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. If the reaction is severe the infusion rate or dose may be reduced.

Tell the doctor if your child has, or has had a severe allergic reaction to cerliponase alfa or any of the other ingredients of Brineura.

Some of the symptoms of an allergic reaction may include:

- 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
- diarrhoea or incontinence.

Talk to the doctor before your child is given Brineura.

- 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 will be determined by the doctor.

  Talk to the doctor if you have any questions about your child's device.
- 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.

Tell the doctor if your child is on a controlled sodium diet.
Each vial of Brineura contains 17.42 mg sodium.

If you have not told the doctor about the above, tell him/her before your child starts receiving Brineura.

Taking other medicines

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

How to use Brineura

How much to use

The recommended dose of Brineura is based upon 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 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 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. The doctor may lower the dose or the speed of the infusion based on the response during the treatment.

The doctor may also give your child additional medicines to treat an allergic reaction or reduce fever before each treatment to reduce side effects that occur during or shortly after treatment.

How long to use Brineura

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

Brineura is a new medicine. It is not known how long it will be of benefit. Children given Brineura will be reviewed regularly to see if they continue to benefit from treatment.

If you miss a dose

If a dose is missed, talk to the doctor or nurse and arrange another visit as soon as possible.

While you are being treated with Brineura

Things you must do

Keep all appointments with the doctor and always discuss anything that is of worry 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 is treating your that they are receiving Brineura.

Things you must not do

Do not stop going to your child's visits for treatment with Brineura without checking with the doctor.

Your child's condition may worsen if they stop receiving Brineura.

Side effects

Tell your doctor or nurse as soon as possible if your child does not feel well while they are receiving Brineura.

All medicines, including Brineura, can have unwanted side effects. Sometimes they are serious, most of the time they are not.

Do not be alarmed by this list of possible side effects. Your child may not experience any of them.

All medicines can have side effects. Your child may need medical treatment if they get some of the side effects. **Ask the doctor to answer any questions you may have.**

Tell the doctor or nurse immediately if your child experiences any of the following:

- fever
- vomiting
- feeling irritable or nervous
- convulsions (seizures)
- inflammation of the brain due to infection of the device; symptoms can include fever, headache, stiff neck, sensitivity to light, nausea, vomiting, and change in mental status (confusion, change in behaviour)
- a slower heart beat
- headache
- symptoms of a cold, such as runny or blocked nose, sneezing and coughing
- pain
- rash or hives
- head dropping (so that the chin drops towards the chest)
- mouth or tongue blisters
- itching, swelling or redness of the eyelid and the white part of the eye
- sickness of the stomach or intestines.

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.

These are the most common side effects of the medicine.

Allergic reactions

Tell your doctor or nurse immediately if you experience any of the following side effects:

- 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
- diarrhoea or incontinence

These can be symptoms of an allergic reaction, which can be serious. If your child has an allergic reaction, the doctor may slow down, or stop the infusion. The doctor may also give additional medicines to manage any allergic reaction. The doctor will decide when Brineura treatment can be restarted.

Tell the doctor if you notice anything else that is 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.

After being treated with Brineura

Storage

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.

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

Product description

What it looks like

Brineura solution is clear to slightly opalescent, colourless to pale yellow; the Brineura solution may occasionally contain thin translucent 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.

Ingredients

Brineura contains 150 mg cerliponase alfa in each vial.

The solution also contains the following 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 any preservative.

Sponsor

BioMarin Pharmaceutical Australia Pty Ltd

119 Willoughby Road

Crows Nest, NSW 2065

Telephone (02) 8520 3255

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

To report adverse events, contact drugsafety@bmrn.com or call BioMarin Australia on 1800 387 876.

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