Brineura® (cerliponase alfa)

Brineura® Co-Pay Assistance Program

The BioMarin Brineura Co-Pay Assistance Program provides financial assistance to eligible patients. It covers out-of-pocket expenses such as co-pay, coinsurance, and deductibles associated with treatment and administration of Brineura® (cerliponase alfa) for eligible participants.

Co-pay eligibility requirements

- Patient must be a resident of one of the 50 US states or Puerto Rico*
- Co-pay support is for on-label patients only
- Offer is valid for commercially insured and cash-paying patients
- Patient must not be insured by a publicly funded health program such as Medicare, Medicaid, Veterans Affairs (VA), the Department of Defense (DoD or TRICARE), or any similar state-funded or federally funded program such as state pharmacy assistance programs

Step 1: Opt in

Caregivers must contact BioMarin RareConnections™ in order to opt into the program and initiate the preliminary screening process. (BioMarin RareConnections™ Patient Registration Form must be submitted, if not previously completed.)

Step 2: Preliminary determination and copay ID generation

Patients deemed eligible via preliminary screening receive a conditional approval letter containing a copay ID number along with instructions on next steps, including the claim submission process.

Step 3: Claim submission and final eligibility determination

- Once the patient is treated with Brineura, the healthcare provider (HCP) submits a claim to the patient's insurance for reimbursement
- The insurance company sends an Explanation of Benefits (EOB) that indicates the cost share for treatment
- In order for a claim to be submitted, the EOB must include the CPT, HCPCS code or drug-specific NDC code associated with the service/product to be reimbursed
- Either the HCP or caregiver may submit the EOB, conditional approval letter, and claim form for processing
- Once a claim is submitted for processing, the final eligibility is determined and communicated to the HCP and the caregiver
- Upon claim approval, a check will be issued to the submitter of the claim, and HCP and caregiver will be notified

Learn More

For questions, more information, and to enroll, contact BioMarin RareConnections™ by phone at 1-866-906-6100 or via email at BRINEURASupport@biomarin-rareconnections.com Monday–Friday, 8AM – 8PM ET.

Please see enclosed Important Safety Information, including important warning for risk of anaphylaxis, and accompanying full Prescribing Information.

*Patients living in certain states (MA or RI) are not eligible for drug administration co-pay support.

BIOMARIN Rare Connections™

INDICATION AND IMPORTANT SAFETY INFORMATION

What is Brineura used for?

Brineura® (cerliponase alfa) is a prescription medication used to slow loss of ability to walk or crawl (ambulation) in pediatric patients with neuronal ceroid lipofuscinosis type 2 (CLN2 disease), also known as tripeptidyl peptidase 1 (TPP1) deficiency.

What is the most important safety information I should know about Brineura?

Severe and life-threatening allergic reactions, including anaphylaxis, can occur during Brineura infusions and up to 24 hours after infusion. These reactions can occur in people receiving Brineura for the first time or in people who have previously received Brineura without having an allergic reaction. Your child's doctor should ensure appropriately trained personnel and equipment for emergency resuscitation (including epinephrine and other emergency medicines) are readily available during your child's Brineura infusion.

Your child's doctor will tell you about the symptoms of life-threatening hypersensitivity reactions, including anaphylaxis and when to seek immediate medical care. Signs of anaphylaxis can include cough, rash, throat tightness, hives, flushing, changes in skin color, low blood pressure, shortness of breath, chest pain, and gastrointestinal symptoms such as nausea, abdominal pain, retching, and vomiting. If a severe allergic reaction (e.g., anaphylaxis) occurs during infusion, the infusion should be stopped immediately, and your child should receive medical attention. Contact your child's doctor or get medical help right away if your child develops any severe symptoms after infusion.

If anaphylaxis occurs, you and your child's healthcare providers should consider the risks and benefits of readministration of Brineura. If the decision is made to readminister Brineura after the occurrence of anaphylaxis, the healthcare providers should ensure appropriately trained personnel and equipment for emergency resuscitation (including epinephrine and other emergency medicines) are readily available during infusion.

Who should not take Brineura?

- Patients with any sign or symptom of acute or unresolved localized infection around the device insertion site (eg, cellulitis or abscess) or suspected or confirmed central nervous system (CNS) infection (eg, cloudy cerebrospinal fluid [CSF] or positive CSF gram stain, or meningitis)
- Patients with active intraventricular access device-related complications (eg, leakage, device failure, or device-related infection, including meningitis)
- Patients with shunts used to drain extra fluid around the brain

Administration: Brineura is only given by infusion into the fluid of the brain (known as an intraventricular injection) and using sterile technique to reduce the risk of infection. An intraventricular access device or port must be in place at least 5 to 7 days prior to the first infusion.

- Prior to administration, it is important to discuss your child's medical history with their doctor
- Tell the doctor if your child is sick or taking any medication and if they are allergic to any medicines
- Brineura is not recommended for use in patients less than 37 weeks post-menstrual age (gestational at birth plus post-natal age) or those weighing less than 2.5kg.

Meningitis and other device-related infections: Intraventricular access device-related infections, including meningitis, were observed with Brineura treatment. Infections required treatment with antibiotics and removal of the access device. If any signs of infection or meningitis occur, contact your child's doctor immediately. The signs and symptoms of infections may not be readily apparent in patients with CLN2 disease.

- Your child's doctor should vigilantly be looking for signs and symptoms of infection, including meningitis, during treatment with Brineura
- Your child's doctor should inspect the scalp and collect samples of your child's CSF prior to each infusion of Brineura, to check for infections and that there is no device failure
- Signs of infection on or around the device insertion site may include redness, tenderness, or discharge

Device-related complications such as device leakage, device failure, leakage of CSF fluid, or bulging of the scalp around or above the intraventricular access device have occurred. In case of intraventricular access device-related complications, Brineura infusions may be discontinued.

Material degradation of the intraventricular access device reservoir was reported after approximately 4 years of administration, which may impact the effective and safe use of the device. During testing such material degradation was recognized after approximately 105 perforations of the intraventricular access device. The intraventricular access device should be replaced prior to 4 years of single-puncture administrations, which equates to approximately 105 administrations of Brineura.

Cardiovascular side effects: Low blood pressure and/or slow heart rate may occur during and following the infusion of Brineura. Contact your child's healthcare provider immediately if these reactions occur. As part of the infusion, the healthcare provider will monitor vital signs (blood pressure, heart rate) before infusion starts, periodically during infusion, and post-infusion, and assess the patient's status after administration to determine if continued observation may be necessary. Additional monitoring is required for patients with a history of cardiac abnormalities. In patients without cardiac abnormalities, regular 12-lead electrocardiogram (ECG) evaluations should be performed every 6 months.

Infusion Associated Reactions (IAR) such as vomiting, seizure, rash, pyrexia, hypersensitivity, and anaphylactic reaction have been observed in patients treated with Brineura. Your child's doctor may prescribe medicines for your child to take 30 to 60 minutes prior to the start of infusion.

The most common side effects reported during Brineura infusions included:

- Fever, problems with the electrical activity of the heart, decreased or increased protein in the fluid of the brain, vomiting, seizures, device-related complications, hypersensitivity, collection of blood outside of blood vessels (hematoma), headache, irritability, increased white blood cell count in the fluid of the brain, device-related infection, slow heart rate, feeling jittery, and low blood pressure.
- The most frequent adverse reactions reported in patients less than 3 years of age treated with BRINEURA were similar to those observed in patients greater than 3 years of age except for hypersensitivity reactions, which were reported in 5 of 8 (63%) in patients less than 3 years of age at baseline compared with 0 of 6 in patients greater than 3 years of age at baseline. The most common manifestations of hypersensitivity were fever and vomiting. Such symptoms resolved over time or with administration of antipyretics, antihistamines and/or corticosteroids. Symptoms of severe hypersensitivity reactions (e.g., anaphylaxis) included rapid heartbeat, throat tightness, coughing, wheezing, trouble breathing, rash, diarrhea, hypotension, increased body temperature and vomiting.

The risk information provided here is not comprehensive. Talk to your healthcare provider to learn more or for medical advice about any side effects.

You may report side effects to BioMarin at 1-866-906-6100.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch or call 1–800-FDA-1088.

Please see accompanying full Prescribing Information with important warning for risk of anaphylaxis or visit www.Brineura.com.

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