BioMarin RareConnections™ Patient Enrollment Form for CLN2 Disease

Fax completed form to **1-888-863-3361** or email to **support@biomarin-rareconnections.com**
Phone: 1-866-906-6100 Hours: M–F 6 AM–5 PM (PST)

PATIENT

First name _____ Last name _____ Birth date _____ Gender ☐ Male ☐ Female

Parent/Caregiver name _____

Home address _____ Suite/Apt _____

City _____ State _____ ZIP _____

Home phone _____ Work phone _____ Cell phone _____

Email _____ Preferred method of contact ☐ Home ☐ Work ☐ Cell ☐ Email

PRESCRIBER

First name _____ Last name _____ Specialty _____

DEA # _____ NPI # _____ License # _____

Medicaid # _____ Tax ID _____ Site Tax ID _____

Primary practice/office location _____

Street address _____ Suite _____

City _____ State _____ ZIP _____

Office contact _____ Phone _____ Fax _____

INSURANCE

Provide copies of all medical and prescription cards—front and back, primary and supplemental coverage.

Primary insurance name _____

Insurance phone number _____

Policyholder name _____

Relationship to patient _____

Group ID _____

Employer _____

Member ID (policy) number _____

Secondary insurance name _____

Insurance phone number _____

Policyholder name _____

Relationship to patient _____

Group ID _____

Employer _____

Member ID (policy) number _____

☐ Patient does not have insurance

Diagnosis code ICD-10: ☐ Neuronal Ceroid Lipofuscinosis, E75.4 ☐ Other _____

SITE OF SERVICE/ ADMINISTRATION

Name of institution _____

Administering physician or infusion provider name _____ Provider's specialty _____

Street address _____ Suite _____

City _____ State _____ ZIP _____

Site contact _____ Email _____

Office phone (and extension) _____ Office fax _____ Site Tax ID _____

Prescriber Declaration

I verify that the patient and prescriber information contained in this enrollment form is complete and accurate to the best of my knowledge and that I have prescribed Brineura® (cerliponase alfa) based on my professional judgment of medical necessity. I authorize BioMarin or its affiliated companies or subcontractors to perform any steps necessary to provide product support for Brineura, including but not limited to insurance verification and case management. I understand that BioMarin may need additional information, and I agree to provide it as needed for purposes of reimbursement.

Prescriber signature _____ Date _____

IMPORTANT SAFETY INFORMATION

Indication

Brineura® (cerliponase alfa) injection for intraventricular use is indicated to slow the loss of ambulation in symptomatic pediatric patients 3 years of age and older with late infantile neuronal ceroid lipofuscinosis type 2 (CLN2), also known as tripeptidyl peptidase 1 (TPP1) deficiency.

Important Safety Information

Brineura is contraindicated in patients with acute intraventricular access device-related complications and with ventriculoperitoneal shunts.

Brineura must only be administered via the intraventricular route and using aseptic technique to reduce the risk of infection. Healthcare professionals should inspect the scalp for skin integrity to ensure the intraventricular access device is not compromised prior to each infusion. Brineura is contraindicated if there are signs of acute intraventricular access device-related complications (e.g., leakage, device failure or signs of device-related infection such as swelling, erythema of the scalp, extravasation of fluid, or bulging of the scalp around or above the intraventricular access device). In case of intraventricular access device complications, discontinue the Brineura infusion and refer to the manufacturer's labeling for further instructions. Routinely send cerebrospinal fluid (CSF) samples for testing to detect subclinical device infections.

Material degradation of the intraventricular access device reservoir may occur after approximately 105 perforations of the intraventricular access device and may require replacement as soon as, or prior to,105 administrations of Brineura.

Monitor vital signs before infusion starts, periodically during infusion, and post-infusion in a healthcare setting. Perform electrocardiogram (ECG) monitoring during infusion in patients with a history of bradycardia, conduction disorder, or with structural heart disease. In patients without cardiac abnormalities, regular 12-lead ECG evaluations should be performed every 6 months.

Hypotension occurred in 2 patients during or up to 8 hours after Brineura infusion. Patients did not require alteration in treatment, and reactions resolved spontaneously or after intravenous fluid administration.

One patient experienced hypoxia 8 hours after Brineura infusion, followed by a low mean arterial pressure at 15 hours post infusion. Symptoms resolved after oxygen administration, airway repositioning, and normal saline infusion. One patient reported decreased oxygen saturation, 45 minutes after starting Brineura, with associated low diastolic blood pressure. Hypoxia resolved after oxygen administration. No treatment was administered for the low diastolic blood pressure, which returned to normal while the patient continued to receive Brineura infusion without change to the infusion rate or dose.

Due to the potential for anaphylaxis, appropriate medical support should be readily available when Brineura is administered. If anaphylaxis occurs, immediately discontinue the infusion and initiate appropriate medical treatment. Observe patients closely during and after the infusion.

Hypersensitivity reactions were reported in 11 patients during or within 24 hours after completion of the Brineura infusion. The signs and symptoms observed concomitantly with hypersensitivity reactions include pyrexia, vomiting, pleocytosis, or irritability. Patients were routinely premedicated with antihistamines with or without antipyretics or corticosteroids, prior to infusion of Brineura.

The management of hypersensitivity reactions should be based on the severity of the reaction and may include temporarily interrupting the infusion, and/or treatment with antihistamines, antipyretics, and/or corticosteroids. If a severe hypersensitivity reaction occurs, immediately discontinue the infusion and initiate appropriate medical treatment.

Brineura has not been studied in pregnancy or lactation.

Safety and effectiveness in pediatric patients below 3 years of age have not been established.

In clinical trials, the most frequently reported adverse reactions (≥8%) were pyrexia, ECG abnormalities, CSF protein decreased, vomiting, seizures, hypersensitivity, CSF protein increased, hematoma, headache, irritability, pleocytosis, device-related infection, bradycardia, feeling jittery, and hypotension.

Seizures were reported in 12 patients and included atonic, generalized tonic-clonic, focal, and absence. Seizures were managed with standard anticonvulsive therapies and did not result in discontinuation of Brineura treatment.

Device-related adverse reactions were reported in 12 patients and included infection, delivery system–related complications, and pleocytosis. Intraventricular access device-related CNS infections were observed in 2 patients. In both cases, antibiotics were administered, the intraventricular access device was replaced, and treatment continued. Device-related complications did not result in discontinuation of Brineura treatment. Other device-related adverse reactions included 1 patient with leakage of the intraventricular access device and 1 with pleocytosis.

Hematoma adverse reactions were reported in 5 patients and presented as hematoma, post procedural hematoma, traumatic hematoma, and subdural hematoma. Hematomas did not require treatment and did not interfere with Brineura infusion.

Anti-drug antibodies (ADAs) were detected in serum (79%) and CSF (33.3%) in patients treated with Brineura. No association was found between serum or CSF ADA titers and incidence or severity of hypersensitivity.

Inform caregivers of the signs and symptoms of anaphylaxis, hypotension, bradycardia, and device-related complications. Instruct them to seek immediate medical care should any of these signs and symptoms occur.

To report SUSPECTED ADVERSE REACTIONS, contact BioMarin Pharmaceutical Inc. at 1-866-906-6100, or FDA at 1-800-FDA-1088, or go to www.fda.gov/medwatch.

Please see accompanying full Prescribing Information, or visit www.Brineura.com.

Brineura® (cerliponase alfa)