

Medical Policy:

Brineura® (cerliponase alfa) Intraventricular infusion

POLICY NUMBER	LAST REVIEW	ORIGIN DATE
MG.MM.PH.227	February 27, 2025	2017

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The treating physician or primary care provider must submit to EmblemHealth, or ConnectiCare, as applicable (hereinafter jointly referred to as “EmblemHealth”), the clinical evidence that the member meets the criteria for the treatment or surgical procedure. Without this documentation and information, EmblemHealth will not be able to properly review the request preauthorization or post-payment review. The clinical review criteria expressed below reflects how EmblemHealth determines whether certain services or supplies are medically necessary. This clinical policy is not intended to pre-empt the judgment of the reviewing medical director or dictate to health care providers how to practice medicine. Health care providers are expected to exercise their medical judgment in rendering appropriate care. Health care providers are expected to exercise their medical judgment in rendering appropriate care.

EmblemHealth established the clinical review criteria based upon a review of currently available clinical information (including clinical outcome studies in the peer reviewed published medical literature, regulatory status of the technology, evidence-based guidelines of public health and health research agencies, evidence-based guidelines and positions of leading national health professional organizations, views of physicians practicing in relevant clinical areas, and other relevant factors). EmblemHealth expressly reserves the right to revise these conclusions as clinical information changes and welcomes further relevant information. Each benefit program defines which services are covered. The conclusion that a particular service or supply is medically necessary does not constitute a representation or warranty that this service or supply is covered and/or paid for by EmblemHealth, as some programs exclude coverage for services or supplies that EmblemHealth considers medically necessary.

If there is a discrepancy between this guideline and a member's benefits program, the benefits program will govern. Identification of selected brand names of devices, tests and procedures in a medical coverage policy is for reference only and is not an endorsement of any one device, test or procedure over another. In addition, coverage may be mandated by applicable legal requirements of a state, the Federal Government or the Centers for Medicare & Medicaid Services (CMS) for Medicare and Medicaid members. All coding and web site links are accurate at time of publication.

EmblemHealth may also use tools developed by third parties, such as the MCG™ Care Guidelines, to assist us in administering health benefits. The MCG™ Care Guidelines are intended to be used in connection with the independent professional medical judgment of a qualified health care provider and do not constitute the practice of medicine or medical advice. EmblemHealth Services Company, LLC, has adopted this policy in providing management, administrative and other services to EmblemHealth Plan, Inc., EmblemHealth Insurance Company, EmblemHealth Services Company, LLC, and Health Insurance Plan of Greater New York (HIP) related to health benefit plans offered by these entities. ConnectiCare, an EmblemHealth company, has also adopted this policy. All of the aforementioned entities are affiliated companies under common control of EmblemHealth Inc.

Definitions

Brineura is indicated to slow the loss of ambulation in symptomatic pediatric patients ≥ 3 years of age with late infantile neuronal ceroid lipofuscinosis type 2 (CLN2), also known as tripeptidyl peptidase 1 (TPP1) deficiency.

Length of Authorization

Coverage will be provided for 12 months and may be renewed.

Dosing Limits [Medical Benefit]

Approve the following dosing (A and B):

- A. 300 mg via intracerebroventricular (ICV) infusion administered once every other week; AND
- B. Each dose is followed by an infusion of intraventricular electrolytes (supplied in the Brineura package).

Max Units (per dose and over time) [HCPCS Unit]:

- 300 billable units (1 kit containing 2 vials) every 14 days

Guideline

I. INITIAL CRITERIA

1. **Late Infantile Neuronal Ceroid Lipofuscinosis Type 2 (CLN2)**. Approve if the patient meets ALL of the following (A, B, C, D and E):
 - A. Patient is ≥ 3 years of age; **AND**
 - B. Patient has a diagnosis of CLN2 disease as confirmed by ONE of the following (i or ii):
 - i. Patient has had a genetic test which confirms the diagnosis of CLN2 disease by two pathogenic mutations in the CLN2 gene; **OR**
 - ii. Patient has had a test which confirms reduced activity of tripeptidyl peptidase 1 (TPP1); **AND**
 - C. Patient has mild to moderate disease documented by a two-domain score of 3 to 6 on the motor and language domains of the Hamburg CLN2 Clinical Rating Scale, with a score of at least 1 in each of these two domains; **AND**
 - D. Patient is ambulatory; **AND**
 - E. Brineura is prescribed by, or in consultation with, a metabolic specialist, geneticist, pediatric neurologist, or a physician specializing in the treatment of neuronal ceroid lipofuscinoses (NCLs).

II. RENEWAL CRITERIA

Coverage can be renewed based on the following criteria:

- A. Patient continues to meet Initial Criteria; **AND**
- B. Absence of unacceptable toxicity from the drug or complications from the device. *Examples of unacceptable toxicity or complications include: meningitis and other intraventricular access device-related infections, intraventricular access device-related complications, severe hypersensitivity reactions including anaphylaxis, severe cardiovascular reactions, etc.*; **AND**
- C. Patient has had a 12-lead ECG evaluation performed within the last 6 months (those with cardiac abnormalities require an ECG during each infusion); **AND**
- D. Patient has responded to therapy compared to pretreatment baseline with stability/lack of decline in motor function/milestones on the Motor domain of the Hamburg CLN2 Clinical Rating Scale [Decline is defined as having an unreversed (sustained) 2-category decline or an unreversed score of 0].

Limitations/Exclusions

Coverage of Brineura is not recommended in the following situations:

1. **Neuronal Ceroid Lipofuscinoses (NCLs) other than late infantile ceroid lipofuscinosis type 2 (CLN2) [e.g., CLN1, CLN3, CLN10, CLN13, and others]**. Brineura has not been studied for NCLs involving mutations in genes other than CLN2
2. Patient must not have acute intraventricular access device-related complications (e.g., leakage, extravasation of fluid, or device failure); **AND**
3. Patient must not have ventriculoperitoneal shunts; **AND**
4. Patient has no sign or symptom of acute, unresolved localized infection on or around the device insertion site (e.g. *cellulitis or abscess*); or a suspected or confirmed CNS infection (e.g. *cloudy CSF, positive CSF gram stain, or meningitis*)

Applicable Procedure Codes

Code	Description
J0567	Injection, cerliponase alfa, 1 mg

Applicable NDCs

Code	Description
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68135-0811-02	Brineura 300mg/10mL Kit
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ICD-10 Diagnoses

Code	Description
E75.4	Neuronal ceroid lipofuscinosis

Revision History

Company(ies)	DATE	REVISION
EmblemHealth & ConnectiCare	2/27/2025	Annual Review: Addition to limitations/exclusions - Patient must not have acute intraventricular access device-related complications (e.g., leakage, extravasation of fluid, or device failure); AND Patient must not have ventriculoperitoneal shunts; AND Patient has no sign or symptom of acute, unresolved localized infection on or around the device insertion site(e.g. cellulitis or abscess); or a suspected or confirmed CNS infection (e.g. cloudy CSF, positive CSF gram stain, or meningitis)
EmblemHealth & ConnectiCare	3/28/2024	Annual Review: Updated dosing limits. Initial Criteria: Updated the following statement to add “by two pathogenic mutations in the CLN2 gene” : “Patient has had a genetic test which confirms the diagnosis of CLN2 disease by two pathogenic mutations in the CLN2 gene;” Added: “Patient has mild to moderate disease documented by a two-domain score of 3 to 6 on the motor and language domains of the Hamburg CLN2 Clinical Rating Scale, with a score of at least 1 in each of these two domains; AND Patient is ambulatory ” Added all renewal criteria
EmblemHealth & ConnectiCare	04/05/2023	Transfer from CCUM Template to Co-Branded medical template. Retired MG.MM.PH.41
EmblemHealth & ConnectiCare	03/23/2022	Annual Revision: No criteria changes
EmblemHealth & ConnectiCare	03/31/2021	Annual Revision: No criteria changes

References

1. Brineura® intraventricular infusion [prescribing information]. Novato, CA: BioMarin; July 2020.