Medical Policy:

Brineura® (cerliponase alfa) Intraventricular infusion

POLICY NUMBER	LAST REVIEW	ORIGIN DATE
MG.MM.PH.227	March 28, 2024	2017

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].

CONDITIONS NOT RECOMMENDED FOR APPROVAL

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

Applicable Procedure Codes

Code	Description
J0567	Injection, cerliponase alfa, 1 mg

Applicable NDCs

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

ICD-10 Diagnoses

Code	Description

E75.4	Neuronal ceroid lipofuscinosis

Revision History

Company(ies)	DATE	REVISION
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.