Brineura (cerliponase alfa)

Effective Date: January 1, 2021	Number: MG.MM.PH.227

Definitions

Brineura is a hydrolytic lysosomal N-terminal tripeptidyl peptidase 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. Brineura is supplied as an injection for intraventricular administration.

Dosage/Administration

Indication	Dose
Late Infantile Neuronal Ceroid Lipofuscinosis Type 2 (CLN2)	– 300 mg via intracerebroventricular (ICV) infusion administered once every other week – Each dose is followed by an infusion of intraventricular electrolytes (supplied in the Brineura package).

Length of Authorization

Coverage will be provided for 12 months.

Guideline

Late Infantile Neuronal Ceroid Lipofuscinosis Type 2 (CLN2)

- The patient is ≥ 3 years of age; **AND**
- The patient has a diagnosis of CLN2 disease as confirmed by ONE of the following:
  - The patient has had a genetic test which confirms the diagnosis of CLN2 disease; **OR**
  - The patient has had a test which confirms reduced activity of tripeptidyl peptidase 1 (TPP1); **AND**
- 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).

Limitations/Exclusions

- Neuronal Ceroid Lipofuscinoses (NCLs) other than late infantile ceroid lipofuscinosis type 2 (CLN2) [e.g., CLN1, CLN3, CLN10, CLN13, and others]
- Coverage is not recommended for circumstances not listed in the Guideline. Criteria will be updated as new published data are available.

Applicable Procedure Codes

J0567	Injection, cerliponase alfa, 1 mg

Applicable NDCs

68135-0811-xx	Brineura single use vial; 150mg/5ml solution

Applicable Diagnosis Codes

E75.4	Neuronal ceroid lipofuscinosis

Revision History

1/1/2021	Criteria apply to Commercial, Medicare, and Medicaid members.

References

1. Brineura® intraventricular injection [prescribing information]. Novato, CA: BioMarin Pharmaceutical Inc.; December 2019.

2. ClinicalTrials.gov. A Phase 1/2 Open-Label Dose-Escalation Study to Evaluate Safety, Tolerability, Pharmacokinetics, and Efficacy of Intracerebroventricular BMN 190 in Patients With Late-Infantile Neuronal Ceroid Lipofuscinosis (CLN2) Disease. https://clinicaltrials.gov/ct2/show/NCT01907087?term=NCT01907087&rank=1. Accessed July 20, 2020.
3. U.S. Food and Drug Administration. FDA News Release: FDA approves first treatment for a form of Batten disease. [Online]. April 27, 2017: https://www.fda.gov/newsevents/newsroom/pressannouncements/ucm555613.htm. Accessed July 20, 2020.

4. ClinicalTrials.gov. A Multicenter, Multinational, Extension Study to Evaluate the Long-Term Efficacy and Safety of BMN 190 in Patients With CLN2 Disease. https://clinicaltrials.gov/ct2/show/NCT02485899?term=NCT02485899&rank=1. Accessed July 20, 2020.