



## Brineura (cerliponase alfa)

**Effective Date: January 1, 2021**

**Number: MG.MM.PH.227**

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### 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)	<ul style="list-style-type: none"><li>– 300 mg via intracerebroventricular (ICV) infusion administered once every other week</li><li>– Each dose is followed by an infusion of intraventricular electrolytes (supplied in the Brineura package).</li></ul>

### Length of Authorization

Coverage will be provided for 12 months.

## Guideline

### Late Infantile Neuronal Ceroid Lipofuscinosis Type 2 (CLN2)

- The patient is  $\geq 3$  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
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### Applicable NDCs

68135-0811-xx	Brineura single use vial; 150mg/5ml solution
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### Applicable Diagnosis Codes

E75.4	Neuronal ceroid lipofuscinosis
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### Revision History

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

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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.