Clinical Policy: Cerliponasa alfa (Brineura)

Reference Number: PA.CP.PHAR.338
Effective Date: 1/18
Last Review Date: 04/18 Revision Log

Description
Cerliponase alfa (Brineura®) is a hydrolytic lysosomal N-terminal tripeptidyl peptidase.

FDA approved indication
Brineura 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.

Policy/Criteria
Provider must submit documentation (which may include office chart notes and lab results) supporting that member has met all approval criteria

It is the policy of PA Health and Wellness® that Brineura is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

A. Late Infantile Neuronal Ceroid Lipofuscinosis Type 2 (must meet all)**:**

1. Diagnosis of late infantile neuronal CLN2;
2. Prescribed by or in consultation with a neurologist;
3. Age ≥ 3 years;
4. Confirmation of CLN2 with both of the following:
   a. TPP1 enzyme activity test demonstrating deficient TPP1 enzyme activity in leukocytes;
   b. Identification of 2 pathogenic mutations *in trans* in the TPP1/CLN2 gene.
5.
6. At the time of request, member does not have ventriculoperitoneal shunts.
7. Dose does not exceed 300 mg administered once every other week as an intraventricular infusion;

Approval duration: 6 months

B. Other diagnoses/indications

1. Refer to PA.CP.PHAR.57 if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized).

II. Continued Therapy

A. Late Infantile Neuronal Ceroid Lipofuscinosis Type 2 (must meet all)**:**

1. Currently receiving medication via of Pennsylvania Health and Wellness benefit or member has previously met initial approval criteria or the Continuity of Care policy (PA.LTSS.PHAR.01) applies;

2. Member is responding positively to therapy per the prescriber's clinical judgement-
3. If request is for a dose increase, new dose does not exceed 300 mg administered once every other week as an intraventricular infusion.

Approval duration: 6 months

B. Other diagnoses/indications (1 or 2)**:**

1. Currently receiving medication via of PA Health and Wellness benefit and documentation supports positive response to therapy or the Continuity of Care policy (PA.LTSS.PHAR.01) applies;.

Approval duration: Duration of request or 6 months (whichever is less); or

2. Refer to PA.CP.PHAR.57 if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized)

III. Appendices/General Information

Appendix A: Abbreviation/Acronym Key

CLN2: ceroid lipofuscinosis type 2
FDA: Food and Drug Administration
TPP1: tripeptidyl peptidase 1

Appendix B: Motor Domain of CLN2 Clinical Rating Scale

- The motor domain of the CLN2 Clinical Rating Scale is scored as follow: walks normally = 3, intermittent falls, clumsiness, obvious instability = 2, no unaided walking or crawling only = 1, immobile, mostly bedridden = 0.
- Decline was defined as having an unreversed (sustained) 2 category decline or an unreversed score of 0 in the motor domain of the CLN2 Clinical Rating Scale.

IV. Dosage and Administration

Indication	Dosing Regimen	Maximum Dose
CLN2	300 mg administered once every other week as an intraventricular infusion followed by infusion of intraventricular electrolytes over approximately 4.5 hours	300 mg every other week

V. Product Availability

Injection: Brineura 150 mg/5 mL (30 mg/mL) solution, two single-dose vials per carton co-packaged with Intraventricular Electrolytes Injection 5 mL in a single-dose vial

VI. References

1. Brineura Prescribing Information. Novato, CA: BioMarin Pharmaceutical Inc.; April 2017. Available at: https://www.brineura.com. Accessed December 19, 2017.
2. Williams RE, Adama HR, Blohm M et al. Management Strategies for CLN2 Disease. Pediatirc Neurology. 2017Apr;(69):102-112. http://dx.doi.org/10.1016/j.pediatrneurol.2017.01.034.
3. Fietz M, AlSayed M, Burke D et al. Diagnosis of neuronal ceroid lipofuscinosis type 2 (CLN2 disease): Expert recommendations for early detection and laboratory diagnosis. Molecular Genetics and Metabolism. 2016 Jul;(119):160-167. http://dx.doi.org/10.1016/j.ymgme.2016.07.011.

Reviews, Revisions, and Approvals	Date	Approval Date
2Q 2018 annual review: age added; modified continued therapy criteria to allow provider to determine presence of positive response instead of requiring no decline or decline or one category of CLN2 Clinical Rating Scale score and added requirement that member has at least a score of at least 1 to ensure continued ambulation; references reviewed and updated.	02.19.18	