CareSource Rxinnovations

PHARMACY POLICY STATEMENT
Marketplace

DRUG NAME	Brineura (cerliponase alfa)
BENEFIT TYPE	Medical
STATUS	Prior Authorization Required

Brineura is an enzyme replacement therapy (ERT); specifically, it is a recombinant form of human tripeptidyl peptidase 1 (TPP1), which is deficient in patients with neuronal ceroid lipofuscinosis type 2 (CLN2) disease. Deficiency of TPP1 enzyme activity results in accumulation of waste that it usually metabolizes in cell lysosomes, particularly in the brain. This build up causes cell death and leads to progressive motor function decline, as well as loss of cognitive and visual functions.
CLN2 is a rare, inherited, neurodegenerative lysosomal storage disease, and one of the more common forms of Batten disease. It is caused by mutation of the CLN2 gene (which produces the TPP1 lysosomal enzyme and is also known as the TPP1 gene). Seizures are commonly the initial symptom, typically with late infantile onset. There is rapid progression to dementia, blindness, and loss of ability to walk or talk, with a greatly shortened life expectancy of about 8-12 years.
Brineura was approved in 2017 and is indicated to slow the loss of ambulation in CLN2 patients. It works by helping to replace the deficient TPP1 enzyme. Brineura must be administered into the cerebrospinal fluid (CSF) by infusion via a surgically implanted intraventricular access device. In clinical trials, patients treated with Brineura had less decline in motor and language function when compared with historical cohorts. There is no cure for the disease itself.

Brineura (cerliponase alfa) will be considered for coverage when the following criteria are met:

Late infantile neuronal ceroid lipofuscinosis type 2 (CLN2), also known as tripeptidyl peptidase 1 (TPP1) deficiency

For **initial** authorization:

1. Member is at least 3 years of age; AND
2. Brineura is prescribed by or in consultation with a pediatric neurologist or a geneticist; AND
3. Member has a diagnosis of late infantile neuronal ceroid lipofuscinosis type 2 (CLN2), also known as tripeptidyl peptidase 1 (TPP1) deficiency, confirmed by BOTH of the following:
   a) Demonstration of deficient TPP1 enzyme activity (in leukocytes, fibroblasts, or dried blood spots)
   b) Identification of causative mutations in each allele of the TPP1/CLN2 gene; AND
4. Member has mild to moderate disease documented by a two-domain score of 3-6 on motor and language domains of the Hamburg Scale, with a score of at least 1 in each of these two domains.
5. **Dosage allowed/Quantity limit:** 300 mg administered once every other week as an intraventricular infusion followed by infusion of intraventricular electrolytes over approximately 4.5 hours. (2 packages per 28 days; each package contains 2 vials of 150 mg/5 mL)

If all the above requirements are met, the medication will be approved for 12 months.

For **reauthorization**:
1. Documentation of slowed loss of ambulation; AND
2. Member does not have an unreversed (sustained) score of 0 on the motor domain of the CLN2 clinical rating scale.

If all the above requirements are met, the medication will be approved for an additional 12 months.

CareSource considers Brineura (cerliponase alfa) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
05/17/2017	New policy for Brineura created.
01/05/2022	Transferred to new template. Updated the J code (effective 1/2019). Updated references. Added specialist requirement. Added diagnostic confirmation criteria. Removed redundancy of not having score of 0. Removed exclusions for other neuro illness, ventilation support, status epilepticus. In renewal section, removed meeting initial criteria (would not all apply), reworded slowed loss of ambulation requirement, added that they can't have score of 0 (patient can no longer walk).
04/17/2024	Removed upper age limit.

References:

1. Brineura [package insert]. Novato, CA: BioMarin Pharmaceutical Inc.; 2020.
2. Mole SE, Schulz A, Badoe E, et al. Guidelines on the diagnosis, clinical assessments, treatment and management for CLN2 disease patients. *Orphanet J Rare Dis*. 2021;16(1):185. Published 2021 Apr 21. doi:10.1186/s13023-021-01813-5
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. *Mol Genet Metab*. 2016;119(1-2):160-167. doi:10.1016/j.ymgme.2016.07.011
4. Schulz A, Ajayi T, Specchio N, et al. Study of Intraventricular Cerliponase Alfa for CLN2 Disease. *N Engl J Med*. 2018;378(20):1898-1907. doi:10.1056/NEJMoa1712649

Effective date: 10/01/2024
Revised date: 04/17/2024