Kansas City

An Independent Licensee of the Blue Cross and Blue Shield Association

Brineura (cerliponase alfa)

Policy Number: 5.02.543
Origination: 10/2017
Last Review: 10/2018
Next Review: 10/2019

Policy

Blue Cross and Blue Shield of Kansas City (Blue KC) will provide coverage for Brineura when it is determined to be medically necessary because the criteria below are met.

When Policy Topic is covered

Brineura is considered medically necessary in the treatment of the following conditions:

Patient must have the following:

Late infantile neuronal ceroid lipofuscinosis type 2 (CLN2)

AND ALL of the following:

1. Diagnosis of CLN2 was confirmed by enzyme assay demonstrating a deficiency of tripeptidyl peptidase 1 (TPP1) activity or by genetic testing

2. Medication is being used to slow the loss of ambulation in symptomatic patients

3. Patients have mild to moderate disease documented by a two-domain score of 3-6 on motor and language domains of the Hamburg CL2 Clinical Rating Scale, with a score of at least 1 in each of these two domains

AND NONE of the following:

1. Acute intraventricular access device-related complications including:
   a. Leakage
   b. Device failure
   c. Device-related infection
2. Ventriculoperitoneal shunt
3. Generalized motor status epilepticus prior to 4 weeks of first dose

When Policy Topic is not covered

Brineura is considered investigational for patients less than 3 or older than 16 years of age and for all other indications.

Considerations

Brineura requires prior authorization through the pharmacy services department and is considered a medical benefit.

Brineura should be administered by, or under the direction of a physician knowledgeable in intraventricular administration. Brineura is administered into the cerebrospinal fluid by infusion via a surgically implanted reservoir and catheter. Administer the first dose 5-7 days after device implantation.

The recommended dosage is 300 mg (10ml) administered once every other week as an intraventricular infusion followed by infusion of Intraventricular Electrolytes over approximately 4.5 hours. The electrolytes are included in the administration kit.

Description of Procedure or Service

Background

Late infantile neuronal ceroid lipofuscinosis type 2 (CLN2), also known as tripeptidyl peptidase 1 (TPP1) deficiency, is a neurodegenerative disease caused by a deficiency of the lysosomal enzyme tripeptidyl peptidase-1 (TPP1), which catabolizes polypeptides in the CNS. Deficiency of the enzyme's activity leads to an accumulation of lysosomal storage materials in the CNS, leading to a progressive decline in motor function. Brineura (cerliponase alfa) is a proenzyme that is taken up by target cells and activated in the lysosome. It subsequently cleaves tripeptides from the N-terminus of proteins in order to slow the loss of ambulation (1).

Regulatory Status

FDA-approved indication: 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 (1).

Brineura is contraindicated in patients with acute intraventricular access device-related complications (e.g., leakage, device failure, or device-related infection). Brineura is also contraindicated in patients with ventriculoperitoneal shunts (1).

In the clinical studies that were conducted the exclusion criteria were children less than 3 years old at enrollment and children 16 years old or older at enrollment (2-3).

Safety and efficacy of Brineura has not been established in pediatric patients under 3 years old (1).

Rationale

Brineura is a hydrolytic lysosomal N-terminal tripeptidyl peptidase that works by decreasing the accumulation of lysosomal storage materials in patients with late infantile neuronal ceroid lipofuscinosis type 2 (CLN2). As a result, Brineura slows the progressive decline in motor function and loss of ambulation (1).

Prior authorization is required to ensure the safe, clinically appropriate and cost-effective use of Brineura while maintaining optimal therapeutic outcomes.

References

1. Brineura [package insert]. Novato, CA: BioMarin Pharmaceuticals Inc.; April 2017.
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. Available at: https://clinicaltrials.gov/ct2/results?term=bmn+190&Search=Search. Accessed January 1, 2017.
3. ClinicalTrials.gov. A Multicenter, Multinational, Extension Study to Evaluate the Long-Term Efficacy and Safety of BMN 190 in Patients With CLN2 Disease. Available at: https://clinicaltrials.gov/ct2/show/NCT02485899?term=bmn+190&rank=3. Accessed January 8, 2017.
4. Fietz M, AlSayed M, Burke D, et al. Diagnosis of neuronal ceroid lipofuscinosis type 2 (CLN2): Expert recommendations for early detection and laboratory diagnosis. Molecular Genetics and Metabolism. 2016 (11): 160-167.

Billing Coding/Physician Documentation Information

J3590	Unclassified biologics
C9014	cerliponase alfa, 1 mg (Code becomes effective for Hospital (OPPS) Billing 1/1/18)
C9399	Unclassified drugs or biologicals (This code should only be used for drugs and biologicals that are approved by the FDA on or after January 1, 2004) (Hospital Outpatient Use ONLY)

Additional Policy Key Words

5.02.543

Policy Implementation/Update Information

10/2017 New policy titled Brineura (cerliponase alfa)
12/2017 Added C9014 and C9399
10/2018 No changes made