Brineura®

Policy Number: HS-328

Original Effective Date: 3/1/2018

Revised Date(s): 3/7/2019; 4/16/2020

APPLICATION STATEMENT

The application of the Clinical Coverage Guideline is subject to the benefit determinations set forth by the Centers for Medicare and Medicaid Services (CMS) National and Local Coverage Determinations and state-specific Medicaid mandates, if any.

DISCLAIMER

The Clinical Coverage Guideline (CCG) is intended to supplement certain standard WellCare benefit plans and aid in administering benefits. Federal and state law, contract language, etc. take precedence over the CCG (e.g., Centers for Medicare and Medicaid Services [CMS] National Coverage Determinations [NCDs], Local Coverage Determinations [LCDs] or other published documents). The terms of an enrollee’s particular Benefit Plan, Evidence of Coverage, Certificate of Coverage, etc., may differ significantly from this Coverage Position. For example, an enrollee’s benefit plan may contain specific exclusions related to the topic addressed in this CCG. Additionally, CCGs relate exclusively to the administration of health benefit plans and are NOT recommendations for treatment, nor should they be used as treatment guidelines. Providers are responsible for the treatment and recommendations provided to the enrollee. The application of the CCG is subject to the benefit determinations set forth by the Centers for Medicare and Medicaid Services (CMS) National and Local Coverage Determinations, and any state-specific Medicaid mandates. Links are current at time of approval by the Medical Policy Committee (MPC) and are subject to change. Lines of business are also subject to change without notice and are noted on www.wellcare.com. Guidelines are also available on the site by selecting the Provider tab, then “Tools” and “Clinical Guidelines.”

BACKGROUND

In April of 2017 the U.S. Food and Drug Administration approved Brineura® (cerliponase alfa) as a treatment for the specific form of Batten disease known as late infantile neuronal ceroid lipofuscinosis type 2 (CLN2, or tripeptidyl peptidase-1 (TPP1) deficiency). The drug is an enzyme replacement therapy and is the first FDA-approved treatment to slow loss of ambulation in symptomatic pediatric patients 3 years of age and older.¹

Brineura® is administered into the cerebrospinal fluid by infusion via a specific surgically implanted reservoir and catheter in the head. It is recommended that the first dose be administered at least 5 to 7 days after device implantation. The drug must be administered under sterile conditions to reduce the risk of infections, and treatment should be managed by a health care professional knowledgeable in intraventricular administration.¹,²

The recommended dose of Brineura® in pediatric patients 3 years of age and older is 300 mg administered once every other week by intraventricular infusion, followed by an infusion of electrolytes. The complete Brineura® infusion takes approximately 4.5 hours. Pre-treatment with antihistamines with or without antipyretics or corticosteroids is recommended 30 to 60 minutes prior to the start of the infusion.¹,²

According to the FDA, the most common adverse reactions in patients treated with Brineura® include fever, ECG abnormalities including bradycardia, hypersensitivity, decrease or increase in CSF protein, vomiting, seizures, hematoma, headache, irritability, increased CSF white blood cell count, device-related infection, feeling jittery and low blood pressure.¹

The FDA reports that the efficacy of Brineura® was established in a non-randomized, single-arm dose escalation clinical study in 22 symptomatic pediatric patients with CLN2 disease and compared to 42 untreated patients with CLN2 disease from an independent historical control group who were at least 3 years old and had motor or language symptoms. Taking into account age, baseline walking ability and genotype, Brineura®-treated patients demonstrated fewer declines in walking ability compared to untreated patients in the natural history cohort.¹
The CLN2 form of Batten disease is a rare inherited disorder that primarily affects the nervous system. In this form of the disease, signs and symptoms typically appear between the ages 2 to 4 years old. The first symptoms are typically language delay, recurrent seizures and ataxia. Affected children may also develop muscle twitches and vision loss. CLN2 disease affects essential motor skills, such as sitting and walking. Individuals with this condition often require the use of a wheelchair by late childhood and typically do not survive past their teens.\(^1\)

Per the Beyond Batten Disease Foundation, while a worldwide incidence rate is difficult to confirm, various studies in different countries suggest rates range from 0.5 – 8 per 100,000 live births. Evidence suggests that juvenile Batten disease is the most common form of pediatric neurodegenerative disease. Approximately 440,000 people in the United States do not have any symptoms but, carry disease-causing mutations in their juvenile Batten disease (CLN3) gene.\(^1\)

**POSITION STATEMENT**

**Applicable To:**
- Medicaid – Kentucky

**Exclusions**

Brineura® is **not considered medically necessary** and not a covered benefit when any of the following apply:

1. Enrollees with acute intraventricular access device-related complications (e.g., leakage, device failure, or device-related infection)
2. Enrollees with ventriculoperitoneal shunts
3. Enrollees 2 years age or younger

**Coverage**

Brineura® is **considered medically necessary** for enrollees with late infantile neuronal ceroid lipofuscinosis type 2 (CLN2) and a covered benefit when all of the following criteria apply:

**Initial Authorization**

1. Patient is 3 years or older; **AND**,  
2. Patient has diagnosis of late infantile neuronal ceroid lipofuscinosis type 2 (CLN2), also known as tripeptidyl peptidase 1 (TPP1) deficiency, as demonstrated by:  
   a. Enzyme activity test indicating deficiency in tripeptidyl peptidase 1 (TPP1) enzyme; **OR**,  
   b. Genetic test indicating pathogenic mutations in each allele of TPP1/CLN2 gene; **AND**,  
3. Physician has assessed the baseline combined Motor plus Language CLN2 Clinical Rating Scale score; **AND**,  
4. Patient is currently ambulatory and symptomatic (as demonstrated by clinical progress notes); **AND**,  
5. Medication was prescribed by a pediatric neurologist or in consultation with a pediatric neurologist who specializes in the treatment of CLN2; **AND**,  
6. Brineura will be administered by, or under the direction of, a physician knowledgeable in intraventricular administration; **AND**,  
7. For patients with history of bradycardia, conduction disorder, or with structural heart disease, electrocardiogram (ECG) monitoring will be performed during infusion.

**Continuation of Care**

1. Patient is 3 years or older; **AND**,  
2. Patient has diagnosis of late infantile neuronal ceroid lipofuscinosis type 2 (CLN2) as demonstrated by:  
   a. Enzyme activity test indicating deficiency in tripeptidyl peptidase 1 (TPP1) enzyme; **OR**,  

Clinical Coverage Guideline
b. Genetic test indicating pathogenic mutations in each allele of TPP1/CLN2 gene; **AND**,  
3. A clinical response defined as lack of decline in the Motor domain of the CLN2 Clinical Rating Scale (decline is defined as a sustained 2-category decline or an unreversed score of 0); **AND**,  
4. Performing of electrocardiogram (ECG) evaluations:  
   a. For patients with history of bradycardia, conduction disorder, or with structural heart disease: during infusion  
   b. For patients without cardiac abnormalities: every 6 months  
5. Medication was prescribed by a pediatric neurologist or in consultation with a pediatric neurologist who specializes in the treatment of CLN2; **AND**,  
6. Brineura will be administered by, or under the direction of, a physician (e.g., pediatric neurologist) knowledgeable in intraventricular administration.  

**Initial authorization:** 6 months  
**Reauthorization:** 12 months

**CODING**

**Covered CPT Codes** – None.  
**Covered HCPCS Code**  
C9014 Injection, cerliponase alfa, 1 mg  
**Covered ICD-10 Code**  
E75.4 Neuronal ceroid lipofuscinosis-late infantile type 2  

Coding information is provided for informational purposes only. The inclusion or omission of a CPT, HCPCS, or ICD-10 code does not imply enrollee coverage or provider reimbursement. Consult the enrollee’s benefits that are in place at time of service to determine coverage (or non-coverage) as well as applicable federal / state laws.

**REFERENCES**


**MEDICAL POLICY COMMITTEE HISTORY AND REVISIONS**

<table>
<thead>
<tr>
<th>Date</th>
<th>Action</th>
</tr>
</thead>
<tbody>
<tr>
<td>4/16/2020</td>
<td>• Approved by MPC. No changes.</td>
</tr>
<tr>
<td>3/7/2019</td>
<td></td>
</tr>
<tr>
<td>3/1/2018</td>
<td>• Approved by MPC. New.</td>
</tr>
</tbody>
</table>

Clinical Coverage Guideline

Original Effective Date: 3/1/2018 - Revised: 3/7/2019; 4/16/2020  
PRO_55378E State Approved 08062020  
©WellCare 2020