Cerliponase Alfa (Brineura)

Descriptive Narrative

Cerliponase Alfa (Brineura) is a hydrolytic lysosomal N-terminal tripeptidyl peptidase which received FDA approval on April 27, 2017 for members with tripeptidyl peptidase-1 deficiency. In essence, this is an enzyme replacement. This disease is also known as neuronal ceroid lipofuscinosis (NCL). The most common juvenile form is known as Batten disease. Symptoms of this disease start with difficulty walking eventually progressing to blindness, immobility, dementia, and death by the late teens or twenties.

This is an inheritable disorder of the nervous system beginning in early childhood resulting in progressive neurologic dysfunction. Occasional autosomal dominant forms of the disease are reported. The incidence of this autosomal recessive disorder is estimated to be 2-4/100,000 live births. There are eight genes identified as resulting in the various categories of NCL. Forms of NCL are classified by onset and include congenital, infantile, late infantile, and adult. Other variants are described.

Cerliponase Alfa (Brineura) is approved for intraventricular infusion to enhance uptake into the brain for members with late infantile neuronal ceroid lipofuscinosis type 2 (CLN2).

Criteria

Initial approval is for 6 months and **ALL** of the following criteria are required for approval of Brineura:

1. Patient has been diagnosed with neuronal ceroid lipofuscinosis type 2 (CLN2); **AND**
2. The patient is at least 3 years of age and there is significant and clinically observable loss of the ability to ambulate; **AND**
3. There are no contraindications to intraventricular access such as a ventriculoperitoneal or ventricular jugular shunt; **AND**
4. A dosage of 300 mg is administered once every other week as an intraventricular infusion followed by an infusion of intraventricular electrolytes over 4.5 hours; **AND**
5. Patient must have a definitive diagnosis of late infantile CLN2 confirmed by deficiency of the lysosomal enzyme tripeptidyl peptidase-1 (TPP1); **AND**

6. Patient has symptomatic disease (e.g., seizures, motor decline, cognitive decline, decreased visual acuity, etc.); **AND**

7. Patient is ambulatory; **AND**

8. Patient must not have ventriculoperitoneal shunts; **AND**

9. Patient must not have acute intraventricular access device-related complications (e.g., leakage, device failure, or device-related infection); **AND**

10. Patients with a history of bradycardia, conduction disorder, or with structural heart disease must have electrocardiogram (ECG) monitoring performed during the infusion; **AND**

11. Baseline documentation of pretreatment motor function/milestones, including but not limited to, the motor domain of a Hamburg CLN2 Clinical Rating Scale, etc.

Renewal approval is for 12 months and **ALL** of the following criteria are required for approval

1. Patient continues to meet the criteria above; **AND**

2. Absence of unacceptable toxicity from the drug or complications from the device. Examples include the following: intraventricular access device leakage or infection, severe hypersensitivity reaction, severe hypotension; etc.; **AND**

3. Patient had a 12-lead ECG evaluation performed within the last 6 months (those with cardiac abnormalities require ECG during each infusion); **AND**

4. Patient has responded to therapy compared to pretreatment baseline with stability/lack of decline in motor function/milestones on validated scale such as the motor domain of the CLN2 Clinical Rating Scale, etc.

**Note:** Decline is 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.

**Coding**

NDC: 68135-811-02
CPT: 96413
J0567

**References**


Development of utilization management criteria may also involve research into other state Medicaid programs, other payer policies, consultation with experts and review by the Medicaid Clinical Advisory Committee (CAC). These sources may not be referenced individually unless they are specifically published and are otherwise applicable to the criteria at issue.

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Signature
C. David Smith, MD

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