Brineura

Description

Brineura (cerliponase alfa)

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: (1)

- Any sign or symptom of acute or unresolved localized infection on or around the device insertion site (e.g. cellulitis or abscess); or suspected or confirmed CNS infection (e.g. cloudy CSF or positive CSF gram stain, or meningitis)
- Any acute intraventricular access device-related complication (e.g., leakage, extravasation of fluid, or device failure)
- Ventriculoperitoneal shunts

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).

Related policies

**Policy**

This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.

Brineura may be considered **medically necessary** in patients 3-16 years of age for the treatment of late infantile neuronal ceroid lipofuscinosis type 2 (CLN2) and if the conditions indicated below are met.

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

**Prior-Approval Requirements**

**Age**  3-16 years of age

**Diagnosis**

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 CLN2 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

Prior – Approval Renewal Requirements

Age 3-16 years of age

Diagnosis

Patient must have the following:

Late infantile neuronal ceroid lipofuscinosis type 2 (CLN2)

AND ALL of the following:
1. Documentation confirming slowed loss of ambulation following first year of treatment

Policy Guidelines

Pre - PA Allowance
None

Prior – Approval Limit
Duration 12 months

Prior – Approval Renewal Limits
Same as above

Rationale

Summary
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

Policy History

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Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on December 6, 2019 and is effective on January 1, 2020.