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 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).

**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 lipofuscinosisis 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 lipofuscinosisis 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
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. 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

Duration 12 months

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

<table>
<thead>
<tr>
<th>Date</th>
<th>Action</th>
</tr>
</thead>
<tbody>
<tr>
<td>July 2017</td>
<td>Addition to PA</td>
</tr>
<tr>
<td>September 2017</td>
<td>Annual review</td>
</tr>
<tr>
<td>November 2018</td>
<td>Annual review and reference update</td>
</tr>
</tbody>
</table>

Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on November 30, 2018 and is effective on January 1, 2019.