Medical Drug Clinical Criteria

Subject:	Brineura (cerliponase alfa)		
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Overview

This document addresses the use of Brineura (cerliponase alfa), a recombinant human tripeptidyl peptidase 1 enzyme replacement therapy for the treatment of late infantile neuronal ceroid lipofuscinosis type 2 (CLN2).

CLN2 is a form of Batten disease, a fatal inherited disorder of the nervous system, and results from a functional reduction in tripeptidyl peptidase 1 (TPP1) which is an enzyme that degrades proteins. Absence of TPP1 results in the accumulation of lysosomal storage materials in the brain and other organs and leads to progressive neurodegeneration and loss of cognitive, motor and visual function.

Brineura is approved to slow the loss of ambulation in individuals with CLN2. Clinical trials included individuals who had mild to moderate disease documented by a two-domain score of 3 to 6 on motor and language domains of the CLN2 Scale, with a score of at least 1 in each of these two domains. The CLN2 Scale was adapted from motor and language domains of the Hamburg rating scale, upon which minor clarifications were made and definitions were elaborated.

Rating Scale/Measure	Description
CLN2 Clinical Rating Scale (Schulz 2018)	The rating scale consists of a Motor Domain and a Language Domain. **Motor Domain** 3=Grossly normal gait, no prominent ataxia, no pathologic falls 2=Independent gait as defined by ability to walk without support for 10 steps; obvious instability and possibly intermittent falls 1=Requires external assistance to walk or can only crawl 0=Can no longer crawl **Language Domain** 3=Apparently normal language that is intelligible and grossly age-appropriate, with no decline noted 2=Language that has recognizable abnormalities but includes some intelligible words; may form short sentences to convey concepts, requests, or needs 1=Language that is hard to understand with few intelligible words 0=No intelligible words or vocalizations Domain scores are added together. Range is 0 to 6.

Brineura has a boxed warning for hypersensitivity reactions, including anaphylaxis, and administration should be in a healthcare setting with appropriate medical monitoring and support measures, including access to cardiopulmonary resuscitation equipment and epinephrine.

Clinical Criteria

When a drug is being reviewed for coverage under a member's medical benefit plan or is otherwise subject to clinical review (including prior authorization), the following criteria will be used to determine whether the drug meets any applicable medical necessity requirements for the intended/prescribed purpose.

Brineura (cerliponase alfa)

Initial requests for Brineura (cerliponase alfa) may be approved for:

I. Individual is equal to or greater than 37 weeks post-menstrual age (gestational age at birth plus post-natal age); **AND**
II. Individual weighs 2.5 kg or more; **AND**
III. Documentation is provided that individuals with a diagnosis of late infantile neuronal ceroid lipofuscinosis type 2 (CLN2) is confirmed by:
    A. Tripeptidyl peptidase 1 (TPP1) deficiency, and documentation is provided; **OR**
    B. Detection of pathogenic mutations in each allele of the TPP1 gene (also known as the neuronal ceroid lipofuscinosis type 2 gene), and documentation is provided; **AND**
IV. Documentation is provided that individual has all of the following on the CLN2 Clinical Rating Scale (Schulz 2018):
    A. Aggregate motor-language domain score of 3 or greater; **AND**
    B. Score of at least 1 on the motor domain; **AND**
    C. Score of at least 1 on the language domain; **AND**
V. Treatment is being given to slow the loss of ambulation.

Continuation requests for Brineura (cerliponase alfa) may be approved for:

I. Individual has a diagnosis of late infantile neuronal ceroid lipofuscinosis type 2 (CLN2); **AND**
II. Documentation is provided that individual has a score of at least 1 on the motor domain of the CLN2 Clinical Rating Scale; **AND**
III. Treatment is being given to slow the loss of ambulation.

Brineura (cerliponase alfa) may not be approved for the following:

I. There are acute intraventricular access device-related complications (such as leakage, device failure, or device-related infection) or ventriculoperitoneal shunts; **OR**
II. Individual has signs or symptoms of acute or unresolved localized infection on or around the device insertion site (such as, cellulitis or abscess), **OR**
III. Individual has suspected or confirmed central nervous system (CNS) infection (such as, cloudy cerebrospinal fluid [CSF], or positive CSF gram stain, or meningitis); **OR**
IV. When the above criteria are not met and for all other indications.

Coding

The following codes for treatments and procedures applicable to this document are included below for informational purposes. Inclusion or exclusion of a procedure, diagnosis or device code(s) does not constitute or imply member coverage or provider reimbursement policy. Please refer to the member's contract benefits in effect at the time of service to determine coverage or non-coverage of these services as it applies to an individual member.

HCPCS

J0567	Injection, cerliponase alfa, 1 mg [Brineura]

ICD-10 Diagnosis

E75.4	Neuronal ceroid lipofuscinosis

Document History

Revised: 09/09/2024
Document History:

- 09/09/2024 – Annual Review: Update use in pediatrics and remove requirement for symptomatic disease based on label update. Add indication to continuation criteria. Coding Reviewed: No changes.
- 09/11/2023 – Annual Review: No changes. Coding Reviewed: No changes.

- 09/12/2022 – Annual Review: No changes. Coding Reviewed: No changes.
- 09/13/2021 – Annual Review: Update criteria to include disease scoring per clinical trials,and add continuation criteria. Administrative update to add documentation. Coding Reviewed: No changes.
- 08/01/2021 – Administrative update to add documentation.
- 09/14/2020 – Annual Review: Wording and formatting changes. Coding Reviewed: No changes.
- 09/09/2019 – Annual Review: Update criteria to add labeled contraindication in those with localized infection around insertion site and those with CNS infections. Minor wording and formatting changes. Coding reviewed: No changes.
- 11/09/2018 – Coding Review: no changes.
- 11/21/2018 – Delete HCPCS codes: J3490 and C9014. Add code J0567.
- 08/17/2018 – Annual Review: Initial review of DRUG.00099. Minor wording and formatting changes.

References

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2. DrugPoints® System [electronic version]. Truven Health Analytics, Greenwood Village, CO. Updated periodically.
3. Lexi-Comp ONLINE™ with AHFS™, Hudson, Ohio: Lexi-Comp, Inc.; Updated periodically.
4. Online Mendelian Inheritance in Man (OMIM) #204500 Ceroid Lipofuscinosis, Neuronal, 2; CLN2. Available from: https://www.omim.org/entry/204500.
5. Schulz A, Ajayi T, Specchio N, et al; CLN2 Study Group. Study of Intraventricular Cerliponase Alfa for CLN2 Disease. *N Engl J Med*. 2018;378:1898-1907. (NCT01090787 and NCT02485899).