Reference number(s)
4714-A

Standard Medicare Part B Management Brineura

Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

Brand Name	Generic Name
Brineura	cerliponase alfa

Indications

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indications[1]

Brineura is indicated to slow the loss of ambulation in pediatric patients with neuronal ceroid lipofuscinosis type 2 (CLN2 disease), also known as tripeptidyl peptidase 1 (TPP1) deficiency.

All other indications will be assessed on an individual basis. Submissions for indications other than those listed in this document should be accompanied by supporting evidence from Medicare approved compendia.

Documentation

The following documentation must be available, upon request, for all submissions: For initial requests, tripeptidyl peptidase 1 (TPP1) enzyme assay or genetic testing results supporting diagnosis.

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Exclusions

Coverage will not be provided for members with any of the following exclusions:

- Dosage of Brineura exceeds 300 mg once every other week.
- Member has acute intraventricular access device-related complications (e.g., leakage, device failure, or device-related infection) or a ventriculoperitoneal shunt.

Coverage Criteria

Neuronal ceroid lipofuscinosis type 2 (CLN2)[1-2]

Authorization of 12 months may be granted for members with CLN2 when all of the following criteria are met:

- Diagnosis of CLN2 was confirmed by enzyme assay demonstrating a deficiency of tripeptidyl peptidase 1 (TPP1) enzyme activity or by genetic testing; and
- Brineura will be administered by, or under the direction of a physician knowledgeable in intraventricular administration.

Continuation of Therapy

All members (including new members) requesting authorization for continuation of therapy must be currently receiving therapy with the requested agent.

Authorization for 12 months may be granted when all of the following criteria are met:

- The member is currently receiving therapy with the requested medication.
- The requested medication is being used to treat an indication listed in the coverage criteria section.
- The requested medication will be administered by, or under the direction of a physician knowledgeable in intraventricular administration.
- The member is receiving benefit from therapy. Benefit is defined as no loss of ambulation or a slowed loss of ambulation from baseline.

Summary of Evidence

The contents of this document were created after examining the following resources: The prescribing information for Brineura.

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- The available compendium
    - National Comprehensive Cancer Network (NCCN) Drugs and Biologics Compendium
    - Micromedex DrugDex
    - American Hospital Formulary Service- Drug Information (AHFS-DI)
    - Lexi-Drugs
    - Clinical Pharmacology
- Diagnosis of neuronal ceroid lipofuscinosis type 2 (CLN2 disease): Expert recommendations for early detection and laboratory diagnosis.

After reviewing the information in the above resources, the FDA-approved indications listed in the prescribing information for Brineura are covered.

Explanation of Rationale

Support for FDA-approved indications can be found in the manufacturer's prescribing information.

Support for using enzyme assays or genetic testing to confirm the diagnosis prior to initiating Brineura to treat neuronal ceroid lipofuscinosis type 2 (CLN2) can be found in a guideline published by Fietz et al. Laboratory diagnosis is from deficient enzyme activity and/or molecular analysis.

References

1. Brineura [package insert]. Novato, CA: BioMarin Pharmaceutical, Inc. July 2024.
2. Fietz M, AlSayed M, Burke, D, et al. Diagnosis of neuronal ceroid lipofuscinosis type 2 (CLN2 disease): Expert recommendations for early detection and laboratory diagnosis. Molecular Genetics and Metabolism. 2016 (11): 160-167.

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