

## SPECIALTY GUIDELINE MANAGEMENT

### BRINEURA (cerliponase alfa)

#### POLICY

##### I. 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 Indication

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 are considered experimental/investigational and not medically necessary.

##### II. DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review: tripeptidyl peptidase 1 (TPP1) enzyme assay or genetic testing results supporting diagnosis.

##### III. EXCLUSIONS

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

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

##### IV. CRITERIA FOR INITIAL APPROVAL

##### **Neuronal ceroid lipofuscinosis type 2 (CLN2)**

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

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

##### V. CONTINUATION OF THERAPY

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for an indication listed in Section IV when both of the following criteria are met:

Reference number
1831-A

- A. The requested medication will be administered by, or under the direction of a physician knowledgeable in intraventricular administration.
- B. Member has experienced no loss of ambulation or a slowed loss of ambulation from baseline.

## VI. 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.