Reference number
1831-A

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 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.

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. CRITERIA FOR INITIAL APPROVAL

Late infantile 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:
1. Diagnosis of CLN2 was confirmed by enzyme assay demonstrating a deficiency of tripeptidyl peptidase 1 (TPP1) enzyme activity or by genetic testing; and
2. Member is 3 years of age or older; and
3. Brineura will be administered by, or under the direction of a physician knowledgeable in intraventricular administration; and
4. Dosage of Brineura will not exceed 300 mg once every other week; and
5. Member does not have acute intraventricular access device-related complications (e.g., leakage, device failure, or device-related infection) or a ventriculoperitoneal shunt.

IV. CONTINUATION OF THERAPY

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for an indication listed in Section III when the following criteria are met:
A. Member has experienced no loss of ambulation or a slowed loss of ambulation from baseline; and
B. Member does not have acute intraventricular access device-related complications (e.g., leakage, device failure, or device-related infection) or ventriculoperitoneal shunts.

V. REFERENCES

1. Brineura [package insert]. Novato, CA: BioMarin Pharmaceutical, Inc.; March 2020.
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.