Brineura® (cerliponase alfa) – Expanded indication

- On July 24, 2024, BioMarin announced the FDA approval of Brineura (cerliponase alfa), 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.
    - Brineura was previously approved for this indication in symptomatic pediatric patients 3 years of age and older with late infantile CLN2 disease.
    - This expanded indication now includes children of all ages with CLN2 disease, regardless of whether they are symptomatic or presymptomatic.
- The approval of Brineura for the expanded indication was based on an open label clinical study in symptomatic and presymptomatic CLN2 patients less than 18 years of age. The study enrolled 14 patients ranging in age from 1 to 6 years at baseline, including 8 patients less than 3 years of age. Patients received Brineura every 2 weeks for 144 weeks.
    - In patients below 3 years of age, none (0%) of the Brineura treated patients had a 2-point decline or score of zero in the Motor score by week 169. Among the 8 treated patients, 7 were matched to 18 untreated patients from the natural history cohort. Among the matched natural history comparators, 11 patients (61%) had an unreversed 2-point decline or score of zero in the Motor score by last assessment.
    - All seven of the treated patients below 3 years of age with a motor score of 3 at baseline remained at a motor score of 3 at the last measured timepoint, which represents grossly normal gait. In this population Brineura treated patients showed a delay in disease onset.
- Brineura carries a boxed warning for hypersensitivity reactions including anaphylaxis.
- The recommended dosage of Brineura in pediatric patients is provided in the table below. The dose is administered once every other week by intraventricular infusion.

Age groups	Brineura dose administered every other week	Volume of Brineura solution
Birth to < 6 months	100 mg	3.3 mL
6 months to < 1 year	150 mg	5 mL
1 year to < 2 years	200 mg (first 4 doses) 300 mg (subsequent doses)	6.7 mL (first 4 doses) 10 mL (subsequent doses)
2 years and older	300 mg	10 mL