

# Neuronal Ceroid Lipofuscinosis Type 2 (CLN2) Disease: Best Practices in Intracerebroventricular Cerliponase Alfa Replacement Therapy

The recombinant human enzyme cerliponase alfa is the first and only approved treatment for CLN2 disease. It also is the first approved enzyme replacement therapy administered via intracerebroventricular infusion. To facilitate safe chronic administration of the treatment, a group of health care professionals from U.S. institutions with experience in cerliponase alfa treatment of children with CLN2 disease developed the following key common and flexible practices.<sup>1</sup>

Key Common Practices <sup>1</sup>	
<b>Dedicated team</b>	<ul style="list-style-type: none"> <li>• Develop multidisciplinary team with dedicated team lead and coordinator</li> </ul>
<b>Process development</b>	<ul style="list-style-type: none"> <li>• Develop cerliponase alfa-specific processes suited to the institution's standards and resources</li> <li>• Promote communication and knowledge sharing with counterparts in other institutions that have cerliponase alfa experience, especially surrounding patient transfers</li> <li>• Provide team training and regular experience to maintain skills</li> </ul>
<b>Choice of ICV device</b>	<ul style="list-style-type: none"> <li>• Select an ICV device that provides stable positioning of port needle base flush with the scalp during infusion</li> <li>• Ensure neurosurgical and infusion teams discuss impact of ICV port design on infusion stability before surgical placement of ICV device</li> </ul>
<b>ICV device access</b>	<ul style="list-style-type: none"> <li>• Familiarize team with ICV device design and access method before first patient</li> </ul>
<b>ICV port replacement</b>	<ul style="list-style-type: none"> <li>• Train team and patient's caregivers to monitor for port CSF leakage</li> <li>• Replace ICV port in the case of infection or device malfunction</li> </ul>
<b>Preparation and administration processes</b>	<ul style="list-style-type: none"> <li>• Certify infusion team in use of strict aseptic techniques</li> <li>• Use premedication and concurrent medication to reduce discomfort and infusion reactions</li> <li>• If removing hair during infusion site preparation, avoid shaving or hair removal cream</li> <li>• Refine the infusion process to minimize the number of connections and disconnections and to reduce infection risk</li> </ul>
<b>Monitoring and management of potential infection</b>	<ul style="list-style-type: none"> <li>• Monitor for scalp infection and CNS infection</li> <li>• Monitor vital signs</li> <li>• In the case of potential infection, delay ICV infusion</li> <li>• In the case of confirmed CSF infection, replace ICV device</li> </ul>

Abbreviations: CNS - central nervous system; CSF - cerebrospinal fluid; ICV - intracerebroventricular



## Flexible Practices<sup>1</sup>

- Adapt size and composition of team to institution-specific needs and patient numbers
- Conduct practice runs of all processes to refine the approach and identify potential problems before implementation
- Antibiotic-impregnated catheters may be used
- Take intraoperative photographs of implanted ICV device to assist team in identifying port dome location
- The recommended 5- to 7-day period<sup>2</sup> between implantation and infusion may be shortened, but postoperative edema can make port access difficult
- Wrapping needed to secure the port-needle connection may be dependent on the ICV device used
- ICV port may be replaced prophylactically after four years of single-puncture administrations (approximately 105 punctures)<sup>2</sup>

Institutions can determine their preferred practice for the following:

- Choice of sterile field techniques and products
- Choice of infusion premedication
- Confirmatory checks before initiating cerliponase alfa thawing (to avoid possible drug wastage owing to patient nonattendance or infusion cancellation for reasons such as potential infection or device malfunction)
- Person responsible for transfer of cerliponase alfa to patient and infusion line priming
- Frequency of monitoring (CSF sampling and vital signs) can vary according to institution
- Duration of post-infusion observation and location (inpatient or outpatient) can depend on patient condition and duration of treatment, and available facilities
- Action regarding cerliponase alfa infusion in the case of patient illness or asymptomatic positive CSF culture should be determined on a case-by-case basis

### References:

1) de los Reyes E, Lehwald L, Augustine EF, Berry-Kravis E, Butler K, Cormier N, Demarest S, Lu S, Madden J, Olaya J, See S, Vierhile A, Wheless JW, Yang A, Cohen-Pfeffer J, Chu D, Leal-Pardinas F, Wang RY. Intracerebroventricular Cerliponase Alfa for Neuronal Ceroid Lipofuscinosis Type 2 Disease: Clinical Practice Considerations From U.S. Clinics. *Pediatric Neurology*. 2020 Sep;110:64-70. doi: 10.1016/j.pediatrneuro.2020.04.018. Epub 2020 May 4.

2) BioMarin Pharmaceutical Inc. Prescribing information: Brineura (cerliponase alfa) injection, for intraventricular use; 2018. Available at: <https://www.brineura.com/wp-content/themes/jupiter-child/assets/pdfs/resources/Brineura-Prescribing-Information.pdf>.



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