State of California—Health and Human Services Agency
Department of Health Care Services

RICHARD FIGUEROA
ACTING DIRECTOR

GAVIN NEWSOM
GOVERNOR

DATE: November 21, 2019, 2019

N.L.: 11-1119
Supersedes N.L.: 07-0718
Index: Benefits

TO: All County California Children's Services Program Administrators, Medical Consultants, and Integrated Systems of Care Division Staff

SUBJECT: Cerliponase Alfa (Brineura) – Authorization Criteria (Revised)

I. PURPOSE

The purpose of this Numbered Letter (N.L.) is to modify California Children's Services (CCS) Program policy regarding the authorization of cerliponase alfa (Brineura), as a treatment for late infantile neuronal ceroid lipofuscinosis, type 2 (CLN2), a form of Batten disease.

II. BACKGROUND

CLN2 disease is a neurodegenerative lysosomal storage genetic disorder characterized by progressive brain and nerve degeneration. This deterioration is caused by a deficiency in the enzyme tripeptidyl peptidase-1 (TPP1), which leads to the accumulation of proteins and lipids in the central nervous system, causing progressive neurological impairment and loss of function. Symptoms include seizures, visual impairment, progressive loss of motor skills, and the loss of the ability to walk and talk. Affected children will lose the ability to walk and talk by six years of age, and the condition is usually fatal between ages of eight to twelve years.

The Food and Drug Administration (FDA) approved cerliponase alfa on April 27, 2017 as an enzyme replacement for TPP1. An open-label clinical trial concluded that CLN2 patients ages three and older treated with cerliponase alfa had 13 times greater odds of decreased loss of motor function and ambulation at 96 weeks, over an independent untreated historical control group.[1] The treatment is not curative.

III. POLICY

A. Effective the date of this letter, cerliponase alfa is a CCS Program benefit when the following criteria are met:

1. For initial authorization:

a. The client has a diagnosis of CLN2, confirmed by genetic testing identifying homozygous or compound heterozygous p.R208 or c.509-1G>C mutations in both TPP1 alleles; or genetic testing and documentation of deficient enzyme (TPP1) levels.

b. The client has mild to moderate disease with a score of at least one in the motor function domain of the CLN2 Clinical Scoring Form (Attachment 1), adapted from the clinical scoring system for late infantile neuronal ceroid lipofuscinoses.[2]

c. The client's care is under the supervision and monitoring of a CCS Program approved Metabolic Special Care Center (SCC) or CCS-paneled specialist with expertise in treating neuronal ceroid lipofuscinoses (NCL).

d. The client is three years of age or older.

e. The request for cerliponase alfa is for the FDA approved indication and dosage.

f. The SCC has submitted a baseline assessment using Attachment 1, described above, for motor/neurologic/visual/language function.

g. Cerliponase alfa administration will be under the direction of individuals with experience in performing intraventricular infusions.

h. Initial authorization shall be for six months.

B. For reauthorization:

1. The SCC or CCS-paneled specialist shall submit documentation showing:

a. There are no unacceptable adverse effects.

b. Client meets initial review criteria in section III.A.1.a – f.

c. There is no decline in the motor function domain of Attachment 1 or the decline is less than predicted if the client were not on cerliponase alfa.

d. Reauthorization shall be for six months unless there is objective clinical improvement, in which case, reauthorization shall be for 12 months.

C. If the criteria described above are not met, but the requesting provider has clinical documentation and/or scientific evidence that may be relevant to the request, the provider may submit this additional documentation to the Integrated Systems of Care Division (ISCD) Medical Director or designee for consideration during the eligibility determination.

IV. POLICY IMPLEMENTATION

A. Cerliponase alfa is not covered by a Service Code Grouping authorization and a separate authorization is needed.

B. For all cerliponase alfa authorizations, requesting providers must submit a CCS Program Service Authorization Request (SAR) as follows:

1. For clients residing in an independent county, requests shall be submitted to the CCS County Office for processing.

2. For clients residing in a dependent county, requests shall be submitted to the Special Populations Unit by email at CCS_Operations@dhcs.ca.gov or via secure RightFax number, (916) 440-5768.

3. For clients residing in a county covered by the Whole Child Model, requests shall be submitted to, and processed by, the managed care plan.

4. Along with applicable diagnosis and procedure codes:

a. ICD-10: E75.4

b. Healthcare Common Procedure Coding System (HCPCS): J0567 (injection, cerliponase alfa, 1mg)

C. For initial authorizations, a CCS Program SAR along with the following must be submitted:

1. A copy of the prescription signed or co-signed by a CCS-paneled physician working at a CCS Program approved SCC.

2. Progress notes from the metabolic SCC documenting laboratory confirmation of TPP1 gene mutations or deficient TPP1 enzyme levels, and disease status; or preferably, a copy of the genetic test result report.

3. Attachment 1 completed by the treating medical specialist or the SCC documenting a motor score of at least one.

D. For continuing authorizations, the SCC shall submit documentation including the client's ambulatory status within the previous six months and document clinical benefit from therapy.

E. If the drug will be provided by an outside pharmacy provider, the pharmacy must bill by National Drug Code (Medi-Cal billing unit is "each" kit).

F. If the drug will not be provided by an outside pharmacy, the CCS program shall authorize billing under the following HCPCS code:

   For dates of service beginning January 1, 2019, CCS shall authorize HCPCS code J0567: injection, cerliponase alfa, 1 mg.

If you have any questions regarding this N.L., please contact the ISCD Medical Director or designee, via email at ISCD-MedicalPolicy@dhcs.ca.gov.

Sincerely,

ORIGINAL SIGNED BY

Roy Schutzengel
Medical Director
Integrated Systems of Care Division

Attachment 1: CLN2 Clinical Scoring Form

[1] Brineura Package Insert, BioMarin Pharmaceutical Inc., Novato, CA; 2017
[2] Steinfeld R, Heim P, von Gregory, H, et al. Late Infantile Neuronal Ceroid Lipofuscinosis: Quantitative Description of the Clinical Course in Patients with CLN2 Mutations. Am Journal of Medical Genetics. 2002; 112:347-354

CLN2 Clinical Scoring Form

(Adapted from the Clinical Scoring System for Late Infantile Neuronal Ceroid Lipofuscinoses)[1]

Full Name:	CCS Case #:	Date Completed:
County:	Age:	Confirmed p.R208* or c.509-1G>C mutations (yes/no)
Documented TPP1 Enzyme Deficiency (yes/no)	Age of onset:	
Other medical condition(s):		

	Clinical Baseline Prior to Doses – center to complete at least one assessment	**Request For Authorization to Continue Doses – center to complete the assessment(s) used for baseline**
Motor Function (circle the most appropriate score):	Date Completed	Date Completed
Walks normally	Score = 3	Score = 3
Frequent falls, clumsiness	Score = 2	Score = 2
No unaided walking or crawling	Score = 1	Score = 1
Immobile, mostly bedridden	Score = 0	Score = 0
Seizures (grand mal) (circle the most appropriate score):	Date Completed	Date Completed
No seizure per 3-month period	Score = 3	Score = 3
1-2 seizures per 3-month period	Score = 2	Score = 2
1 seizure per month	Score = 1	Score = 1
>1 seizure per month	Score = 0	Score = 0
Visual Function (circle the most appropriate score):	Date Completed	Date Completed
Recognizes desirable object	Score = 3	Score = 3
Grabbing for objects	Score = 2	Score = 2
Reacts to light	Score = 1	Score = 1
No reaction to visual stimuli	Score = 0	Score = 0
Language (circle the most appropriate score):	Date Completed	Date Completed
Normal (individual maximum)	Score = 3	Score = 3
Has become recognizably abnormal	Score = 2	Score = 2
Hardly understandable	Score = 1	Score = 1
Unintelligible or no language	Score = 0	Score = 0

Adverse effects of cerliponase alfa:	
Notes:	
Form Completed By (Name/Title):	Date Completed:
Special Care Facility Name:	

To be completed by CCS staff			
Approved:	Denied:	Reason for Denial:	Date:
Reviewed By (ISCD staff):			

[1] Steinfeld R, Heim P, von Gregory, H, et al. Late Infantile Neuronal Ceroid Lipofuscinosis: Quantitative Description of the Clinical Course in Patients With CLN2 Mutations. Am Journal of Medical Genetics. 2002; 112:347-354