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

JENNIFER KENT
Director

EDMOND G. BROWN JR
Governor

DATE: July 10, 2018

N.L.: 07-0718
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Index: Benefits

TO: ALL COUNTY CALIFORNIA CHILDREN SERVICES (CCS) PROGRAM ADMINISTRATORS, MEDICAL CONSULTANTS, INTEGRATED SYSTEMS OF CARE DIVISION STAFF, AND THE GENETICALLY HANDICAPPED PERSONS PROGRAM (GHPP)

SUBJECT: CERLIPONASE ALFA (BRINEURA)

I. PURPOSE

The purpose of this Numbered Letter (N.L.) is to modify 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 accumulation of proteins and lipids in the central nervous system, causing progressive neurological impairment and loss of function. Symptoms include seizures, visual impairment, and progressive loss of motor skills, ability to walk and talk. Affected children will lose ability to walk and talk by six years of age and the condition is usually fatal by age eight to twelve years of age.

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 Brineura, had 13 times greater odds of decreased loss of motor function and ambulation at 96 weeks, over an independent untreated historical control group[1]. Treatment is not curative.

[1] Brineura Package Insert, BioMarin Pharmaceutical Inc., Novato, CA; 2017

III. POLICY

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

1. The client meets the CCS Program residential, financial and medical eligibility criteria;

2. 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;

3. The client has mild to moderate disease with a score of at least one in the motor domain of the CLN2 Clinical Scoring Form, adapted from the Clinical Scoring System for Late Infantile Neuronal Ceroid Lipofuscinoses[2].

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

5. The client is three years of age or older; and

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

7. Center has submitted a baseline assessment of motor/neurologic/visual/language function, using the modified CLN2 Clinical Scoring Form, described above;

8. Brineura administration will be under the direction of individuals with experience in performing intraventricular infusions;

9. For reauthorization, center shall submit documentation substantiating the following:

   a. There are no unacceptable adverse effects.

   b. Client meets initial review criteria.

   c. Documented efficacy (no decline in the motor domain of the CLN2 Clinical Scoring Form).

[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

B. Initial authorization shall be for 6 months.

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

IV. POLICY IMPLEMENTATION

A. Cerliponase alfa (Brineura) requires a separate authorization.

B. Requesting CCS Program providers must submit to their local county CCS Program office or Special Populations Authorization Unit:

1. A CCS Program Service Authorization Request with applicable diagnosis and procedure codes which may include:

ICD-10 E75.4

HCPCS C9014 Injection, cerliponase alfa, 1 mg.

2. A copy of the prescription from the CCS Program approved SCC; and

3. Progress notes from the metabolic SCC documenting laboratory confirmation of TPP1 gene mutations or deficient TPP1 enzyme levels, and disease status; and

4. CLN2 Clinical Scoring Form, completed by the treating medical specialist or SCC documenting motor score of at least “one”.

C. All requests shall be reviewed by a CCS Program Medical Consultant before authorization of cerliponase alfa.

D. For continuing authorizations, the center must update the client’s ambulatory status and document clinical benefit from therapy, at least annually.

E. If the drug will be supplied 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, a HCPCS code may be issued to the special care center. Effective for dates of service beginning January 1, 2018, CCS Programs will authorize temporary HCPCS code, C9014, until a permanent HCPCS code is released. 1 unit C9014 = Injection, cerliponase alfa, 1mg;

G. The State CCS Program Medical Director or designee will review exceptions on a case-by-case basis.

If you have any questions regarding this N.L., please contact Jill Abramson, M.D., M.P.H, by telephone at (916) 713-8388 or by e-mail at Jill.Abramson@dhcs.ca.gov.

Sincerely,

ORIGINAL SIGNED BY

Sarah Eberhardt-Rios, Division Chief
Integrated Systems of Care Division

Enclosure: CLN2 Clinical Scoring Form

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	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