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

JENNIFER KENT
DIRECTOR

EDMUND G. BROWN JR.
GOVERNOR

DATE: October 10, 2017

N.L.: 10-1017
Index: Benefits

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

SUBJECT: CERLIPONASE ALFA (BRINEURA)

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

I. BACKGROUND

CLN2 disease is a rare and fatal lysosomal storage genetic disorder causing progressive brain and nerve degeneration. This deterioration is caused by a deficiency in the enzyme, tripeptidyl peptidase-1 (TPP1), which leads to a build-up 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 is usually fatal by age eight to twelve years of age.

The Federal 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 the drug had a 13 times greater odds on slowing the loss of motor function and ambulation in CLN2 patients aged three and older over an independent historical control group[1]. Treatment is not curative.

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

II. POLICY

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

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

B. The client has a diagnosis of CLN2, confirmed by genetic testing and enzyme (TPP1) levels;

C. The client's care is under the supervision and monitoring of a CCS Program approved Metabolic Specialty Care Center (SCC);

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

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

III. POLICY IMPLEMENTATION

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

B. Requesting CCS Program providers must submit:

1. A CCS Program Service Authorization Request (SAR) to their local county CCS Program office or Dependent County Regional Office along with:

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

   b. Progress notes from the metabolic special care center documenting laboratory confirmation of CLN2, TPP1 enzyme levels, and disease status.

C. All requests shall be reviewed by a CCS Program Medical Director or designee before authorization of cerliponase alfa;

D. For continuing authorization, the center must submit documentation annually, showing:

1. Updated TPP1 enzyme levels; and

2. The client's ambulatory status;

E. Pharmacy providers must bill by National Drug Code (NDC) (Medi-Cal billing unit is “each” kit). Non-pharmacy providers should use J3590 until a specific Healthcare Common Procedure Coding System (HCPCS) code is established;

F. 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) 327-2108 or by e-mail at Jill.Abramson@dhcs.ca.gov.

Sincerely,

Originally Signed by Jacey Cooper

Jacey Cooper, Acting Chief
Integrated Systems of Care Division