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

JENNIFER KENT
Director

EDMOND G. BROWN JR
Governor

DATE: February 22, 2017

N.L.: 02-0217
Index: Benefits

TO: ALL COUNTY CALIFORNIA CHILDREN SERVICES (CCS) PROGRAM ADMINISTRATORS, MEDICAL CONSULTANTS, STATE SYSTEMS OF CARE DIVISION STAFF

SUBJECT: ETEPLIRSEN (EXONDYS 51™)

The purpose of this Numbered Letter (N.L.) is to establish CCS* Program policy regarding the authorization of Eteplirsen (Exondys 51™), as a treatment for Duchenne muscular dystrophy (DMD), for CCS Program clients with confirmed mutation of the dystrophin gene amenable to exon 51 skipping.

BACKGROUND

DMD is a genetic disorder causing progressive muscle deterioration and weakness. This deterioration is caused by the absence or deficient levels of dystrophin protein, which maintains intact muscle cells. DMD primarily affects skeletal, diaphragm, and heart muscle. DMD occurs in about 1 in 3600 male infants worldwide and about 13% of DMD patients have the genetic mutation of the dystrophin gene amenable to exon 51 skipping. Occurrence in females is rare. Symptoms appear between ages 3 and 5 and progressively worsen over time. Affected individuals gradually lose their ability to perform daily activities and are usually wheelchair bound by adolescence and ventilator dependent by their 20s or 30s.

Eteplirsen is the first treatment for DMD approved in the United States that targets dystrophin deficiency; it received accelerated approval, September 19, 2016, by the U.S. Food and Drug Administration (FDA). It is approved as a once weekly intravenous infusion of 30 milligrams per kilogram for the treatment of DMD in patients who have a confirmed mutation in the DMD gene that is amenable to exon 51 skipping (please see Sarepta product insert about Eteplirsen for detailed prescribing information.)**

* Muscular dystrophy is not a GHPP eligible condition.
** Since eteplirsen is under accelerated approval, continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

I. POLICY

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

A. CCS Program client identified with DMD with exon 51 amenable dystrophin gene mutation, documented by genetic test(s), whose care is under the supervision and monitoring of a CCS Program approved Specialty Care Center (SCC) Neurologist, and;

B. For a client four years of age or older, and;

C. CCS Program SCC Center has provided the following information (using the attached Eteplirsen (Exondys 51™) Patient Request Form):

1. Documentation of % forced vital capacity (FVC) ≥ 30% or

2. Brooke Score ≤ 5 (e.g. some useful hand function present for use of adaptive technology)

3. Baseline urinalysis showing absence of proteinuria

4. Baseline BUN (blood urea nitrogen)/Cr (serum creatinine)

5. Baseline 6MWT (6-minute walk test)

D. Request is for the FDA approved dosage only

E. Completed Eteplirsen (Exondys 51™) Patient Request Form

II. POLICY IMPLEMENTATION

A. Eteplirsen (Exondys 51™) requires separate authorization.

B. Requesting CCS Program providers must submit a CCS Program Service Authorization Request (SAR) to their local county CCS program office or Dependent County Regional Office along with the required documentation from the CCS program approved SCC: a copy of the prescription, and a copy of the Eteplirsen (Exondys 51™) Request Form.

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

D. The local county CCS program office or Dependent County Regional Office must submit the SAR and the Eteplirsen (Exondys 51™) Request Form to the DHCS Systems of Care Division Operations by fax at (916) 440-5768 or e-mail at CCS_Operations@dhcs.ca.gov.

E. Initial authorization shall be for a 6-month trial.

F. Extension of the initial authorization can occur every 6 months. Requesting CCS Program providers shall submit a SAR and a new Eteplirsen (Exondys 51™) Request Form with each 6 month Eteplirsen refill request.

G. CCS Program clients with % FVC < 30% and Brooke Score of 6 will not be granted SAR authorizations because; at the time of this N.L. there is insufficient evidence of efficacy in the specified population.

H. Exceptions will be reviewed on a case-by-case basis by the State CCS Program Medical Director or designee.

If you have any questions regarding this N.L., please contact Dr. Jill Abramson via e-mail at Jill.Abramson@dhcs.ca.gov or by telephone at (916) 327-2108.

Sincerely,

ORIGINAL SIGNED BY PATRICIA MCCLELLAND

Patricia McClelland, Chief
Systems of Care Division

Attachment: Eteplirsen (Exondys 51) Request Form

Eteplirsen (Exondys 51) Request Form

Full Name		CCS Case #		Date Completed	
County		Age		Exon 51 Skip Amenable Mutation (Yes/No)	

I. Clinical Baseline

a. BMI/Weight		
b. FVC (Forced Vital Capacity) %		Date Completed
c. Brooke score		Date Completed
d. 6MWT (6-minute walk test)		Date Completed
e. Urinalysis shows absence of proteinuria (Y/N)		Date Completed
f. BUN		Date Completed
g. Creatinine		Date Completed
h. Notes		

i. Form Completed By (Name/Title)	j. Special Care Facility Name	k. Date Completed

II. Request For Reauthorization After Exondys 51 Trial

a. FVC %		Date Completed
b.6MWT		Date Completed
c. List drug changes after start of Exondys 51		
d. Urinalysis shows absence of proteinuria (Y/N)		Date Completed
e. BUN		Date Completed
f. Creatinine		Date Completed
g. Any adverse reactions with Exondys 51		
h. Additional Details		

i. Form Completed By (Name/Title)	j. Special Care Facility Name	k. Date Completed

To be completed by CCS staff

Approved ☐ Denied ☐ Reason for Denial: Date:

Reviewed By (SCD staff)