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

JENNIFER KENT
DIRECTOR

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GOVERNOR

DATE: July 24, 2017

N.L.: 08-0717
Index: Benefits

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

SUBJECT: DEFLAZACORT (EMFLAZA™)

I. PURPOSE

The purpose of this Numbered Letter (N.L.) is to establish the CCS Program policy regarding the authorization of deflazacort (Emflaza™), as a treatment for Duchenne muscular dystrophy (DMD).

II. 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 one in 3,600 male infants worldwide with estimated incidence of one in 5,000 male births in the United States. 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.

Deflazacort is a glucocorticoid that first launched in 1985 in Europe, and has been used as an anti-inflammatory, and immunosuppressant in countries outside of the United States since the mid-1990s. On February 9, 2017, it received approval by the Food and Drug Administration (FDA) for the treatment of the signs and symptoms of DMD.

Compared to placebo, corticosteroids including prednisone and deflazacort have been shown to improve strength and pulmonary function[1] in DMD. Compared to prednisone, deflazacort may be associated with less weight gain over the first years of treatment, and greater risk of cataracts.[2,3]

III. POLICY

Effective the date of this letter, deflazacort 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 Duchenne muscular dystrophy, confirmed by genetic testing,

C. The client's care is under the supervision and monitoring of a CCS Program approved Special Care Center (SCC) neurologist or physiatrist,

D. The client is five years of age or older,

E. The request for deflazacort is for the FDA approved indication and dosage,

F. The client's weight or body mass index is stable or improved while on deflazacort

IV. POLICY IMPLEMENTATION

A. Deflazacort (Emflaza™) requires a separate authorization,

B. Requesting 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 neurologist or physiatrist,

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[1] Griggs RC, Miller JP, Greenberg CR, et al. Efficacy and safety of deflazacort vs prednisone and placebo for Duchenne muscular dystrophy. Neurology. 2016;87(20):2123-2131.
[2] Bello L, Gordish-Dressman H, Morgenroth LP, et al. Prednisone/prednisolone and deflazacort regimens in the CINRG Duchenne Natural History Study. *Neurology.* 2015;85(12):1048-1055.
[3] Gloss D, Moxley RT, Ashwal S, et al. Practice guideline update summary: corticosteroid treatment of Duchenne muscular dystrophy: Report of the Guideline Development Subcommittee of the American Academy of Neurology. *Neurology.* 2016;86:465-472.

b. Progress notes from the neurology or rehabilitation SCC documenting laboratory confirmation of DMD and disease status.

c. Documentation in medical record or separate document from SCC physician justifying use of deflazacort instead of prednisone.

C. County and regional office staff shall pend a service authorization request when the required documentation has been submitted.

D. All requests shall be reviewed by a CCS Program State Medical Consultant in consultation with a local county CCS program Medical Director or designee before authorization of deflazacort.

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

1. Monitoring of weight and height or use of tools to measure body mass composition at least annually.

2. Documentation of client's ambulatory status.

3. Documentation of ophthalmology follow-up annually (minimum) for surveillance and medical follow-up of potential cataracts.

4. Report by registered dietician.

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, Chief, Medical Policy & Consultation Section, at (916) 327-2108 or via e-mail at Jill.Abramson@dhcs.ca.gov.

Sincerely,

ORIGINAL SIGNED BY PATRICIA MCCLELLAND

Patricia McClelland, Chief
Systems of Care Division