Blue Cross Blue Shield Blue Care Network of Michigan

Nonprofit corporations and independent licensees of the Blue Cross and Blue Shield Association

Medical benefit drug policies are a source for BCBSM and BCN medical policy information only. These documents are not to be used to determine benefits or reimbursement. Please reference the appropriate certificate or contract for benefit information. This policy may be updated and therefore subject to change.

Effective Date: 08/10/2023

Elevidys™ (delandistrogene moxeparvovec-rokl)

HCPCS: J1413

Policy:

Requests must be supported by submission of chart notes and patient specific documentation.

A. Coverage of the requested drug is provided when all the following are met:
   a. Coverage of the requested drug is considered investigational/experimental for all indications due to insufficient evidence of a clinical benefit
      i. BCBSM and BCN are awaiting the results of ongoing clinical trials to provide evidence of a clinical benefit

***Note: Coverage and approval duration may differ for Medicare Part B members based on any applicable criteria outlined in Local Coverage Determinations (LCD) or National Coverage Determinations (NCD) as determined by Center for Medicare and Medicaid Services (CMS). See the CMS website at http://www.cms.hhs.gov/. Determination of coverage of Part B drugs is based on medically accepted indications which have supported citations included or approved for inclusion determined by CMS approved compendia.

Background Information:

- Duchenne muscular dystrophy (DMD) is a rare, life-limiting, progressive childhood disease that affects 1 in 3,500 – 5,000 live male births. It is characterized by progressive muscle weakness and wasting due to the absence of dystrophin protein that causes degeneration of skeletal and cardiac muscle. Affected individuals are unable to run and jump properly due to proximal muscle weakness of the leg and pelvic muscles. DMD occurs as a result of mutations in the dystrophin gene, located on the X-chromosome, which normally functions to generate dystrophin, a structural protein of muscle cells. Mutations in the dystrophin gene lead to an absence of or a defect in dystrophin protein resulting in the progressive symptoms seen in DMD patients.

- Glucocorticosteroids are the main pharmacologic treatment option used in DMD. The 2018 treatment guidelines for DMD support the use of glucocorticosteroids as they are the only medication currently available to slow the decline in muscle strength and function which in turn reduces the risk of scoliosis and stabilizes pulmonary function. Trials show muscle strength is improved when treated with prednisone at a dose of 0.75 mg/kg daily for up to 6 months. The goal of glucocorticoids in an ambulatory patient is the preservation of ambulation and the minimization of later cardiac, respiratory, and orthopedic complications. Continued treatment after the patient becomes non-ambulatory has shown reduction in the risk of progressive scoliosis and stabilization of pulmonary function tests. However, it is

important to note that glucocorticosteroids are not able to induce the production of dystrophin-like proteins and therefore do not alter or impact the underlying cause of DMD.

- The exon skipping therapies, including Exondys 51™, Vyondys 53™, Viltepso™, and Amondys 45™, are disease modifying therapies for the treatment of DMD. All were FDA approved under the accelerated approval pathway using a surrogate endpoint of increase in dystrophin in skeletal muscle. There is no evidence the small observed increase in dystrophin from use of these therapies results in a clinically meaningful benefit. Therefore, establishment of a clinical benefit is needed in on-going clinical trials for the exon skipping therapies to not be considered experimental/investigational.

- Elevidys is an adeno-associated virus vector-based gene therapy indicated for the treatment of ambulatory pediatric patients aged 4 through 5 years with Duchenne muscular dystrophy (DMD) with a confirmed mutation in the DMD gene.

- Similar to the exon skipping therapies, Elevidys was FDA approved under accelerated approval based on expression of Elevidys micro-dystrophin in skeletal muscle observed in patients treated with Elevidys. There is no evidence the small observed increase in dystrophin results in a clinically meaning benefit. Therefore, establishment of a clinical benefit is needed in on-going clinical trials.

- There is an ongoing Phase III, randomized, double-blind, placebo-controlled confirmatory trial (EMBARK) evaluating the efficacy and safety of Elevidys in 120 patients with DMD. The primary endpoint will be change in North Star Ambulatory Assessment total score from baseline to week 52 compared to placebo. The trial will include stratification of participants by age and baseline NSAA with a minimum of 50% of enrolled patients being age 4 – 5. Patients will be included if they are on a stable daily dose of oral corticosteroids and have rAAVrh74 antibody titers less than 1:400. Study results should be available mid to late 2023.

- Based on the current information available, there is insufficient evidence that Elevidys provides a clinical benefit in patients with DMD. Therefore, demonstration of a clinical benefit is warranted in on-going clinical trials.

References:

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12. Clinicaltrials.gov. A phase 3 multinational, randomized, double-blind, placebo-controlled systemic gene delivery study to evaluate the safety and efficacy of SRP-9001 in subjects with duchenne Muscular dystrophy (EMBARK) (NCT05096221). Available at: https://clinicaltrials.gov/ct2/show/NCT05096221?intr=%22SRP-9001%22+OR+%22Delandistrogene+Moxeparvovec%22+OR+%22rAAVrh74.MHCK7.micro-dystrophin%22&draw=1&rank=3. Accessed on June 26, 2023.
13. National Organization for Rare Diseases. Duchenne muscular dystrophy. 2021 March 25. Available at: https://rarediseases.org/rare-diseases/duchenne-muscular-dystrophy/. Accessed on June 26, 2023.

<table>
<tr><th colspan="3">Policy History</th></tr>
<tr><th>#</th><th>Date</th><th>Change Description</th></tr>
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<tr><td>1.2</td><td>Effective Date: 07/10/2023</td><td>UM medical management system update for MAPPO and BCNA
<table><tr><th>Line of Business</th><th>PA Required in Medical Management System (Yes/No)</th></tr><tr><td>BCBS</td><td>Yes</td></tr><tr><td>BCN</td><td>Yes</td></tr><tr><td>MAPPO</td><td>Yes</td></tr><tr><td>BCNA</td><td>Yes</td></tr></table></td></tr>
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<table><tr><th>Line of Business</th><th>PA Required in Medical Management System (Yes/No)</th></tr><tr><td>BCBS</td><td>Yes</td></tr><tr><td>BCN</td><td>Yes</td></tr><tr><td>MAPPO</td><td>No</td></tr><tr><td>BCNA</td><td>No</td></tr></table></td></tr>
<tr><td>1.0</td><td>Effective Date: 04/06/2023</td><td>Preliminary drug review
<table><tr><th>Line of Business</th><th>PA Required in Medical Management System (Yes/No)</th></tr><tr><td>BCBS</td><td>No</td></tr><tr><td>BCN</td><td>No</td></tr><tr><td>MAPPO</td><td>No</td></tr><tr><td>BCNA</td><td>No</td></tr></table></td></tr>
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** The prescribing information for a drug is subject to change. To ensure you are reading the most current information it is advised that you reference the most updated prescribing information by visiting the drug or manufacturer website or http://dailymed.nlm.nih.gov/dailymed/index.cfm.*

Blue Cross Blue Shield/Blue Care Network of Michigan
Medication Authorization Request Form

Blue Cross Blue Shield Blue Care Network of Michigan

Nonprofit corporations and independent licensees of the Blue Cross and Blue Shield Association

This form is to be used by participating physicians to obtain coverage for **drugs covered under the medical benefit**. For commercial members only, please complete this form and submit via fax to 1-877-325-5979. If you have any questions regarding this process, please contact BCBSM Provider Relations and Servicing or the Medical Drug Helpdesk at 1-800-437-3803 for assistance.

PATIENT INFORMATION	PHYSICIAN INFORMATION
Name	**Name**
ID Number	**Specialty**
D.O.B. ☐Male ☐Female	**Address**
Diagnosis	**City /State/Zip**
Drug Name	**Phone/Fax: P**: ( ) - **F**: ( ) -
Dose and Quantity	**NPI**
Directions	**Contact Person**
Date of Service(s)	**Contact Person Phone / Ext.**

STEP 1: DISEASE STATE INFORMATION

1. Is this request for: ☐ Initiation ☐ Continuation *Date patient started therapy:* _____
2. Administered by patient or a medical professional? ☐ patient (self) ☐ health care professional (physician, nurse, etc.)
3. Site of administration? ☐ Provider office/Home infusion ☐ Other: _____
   ☐ Hospital outpatient facility (go to #4) *Reason for Hospital Outpatient administration:* _____
   ☐ Hospital inpatient facility for Car-T therapy only (for example: Kymriah, Yescarta, or Tecartus) (go to #5)
4. Please specify location of administration if hospital outpatient infusion: _____
5. Please specify location of administration if hospital inpatient infusion: _____
6. Please provide the NPI number for the place of administration: _____
7. **Initiation AND Continuation of therapy:**
   a. What is the patient's diagnosis? _____
   b. What other medication has the patient received for their condition? Please list_____
      i. Please describe the response to previous therapies:
      _____
   c. Will the patient be receiving any other treatment for the listed condition while on this medication? Please list:
      _____
   d. Please list any labs values important for diagnosing or monitoring this patient's condition:
      _____
8. **Continuation of therapy:**
   a. Has the patient progressed while on this medication? ☐ yes ☐ no
   b. How has the patient's condition changed while on this medication?
      ☐ Improved: Please describe: _____
      ☐ Stable: please describe: _____
      ☐ Worsened; Please describe: _____
      ☐ Other; Please describe: _____

Chart notes are required for the processing of all requests. Please add any other supporting medical information necessary for our review **(required)**

Coverage will not be provided if the prescribing physician's signature and date are not reflected on this document.

☐ Request for expedited review: I certify that applying the standard review time frame may seriously jeopardize the life or health of the member or the member's ability to regain maximum function

Physician's Name **Physician Signature** **Date**

Step 2: Checklist	☐ Form Completely Filled Out ☐ Provide chart notes	☐ Attach test results
Step 3: Submit	**By Fax: BCBSM Specialty Pharmacy Mailbox 1-877-325-5979**	**By Mail: BCBSM Specialty Pharmacy Program P.O. Box 312320, Detroit, MI 48231-2320**

7/26/2018, 9/18/2018; 1/31/2020; 3/17/2020; 8/9/2021