DR.016.C ELEVIDYS® (Delandistrogene moxeparvovec-rokl)

Original Implementation Date : 07/01/2024
Version [C] Date: 10/16/2025
PARP Approval Date: 01/13/2026
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PRODUCT VARIATIONS

This policy applies to all Jefferson Health Plans/Health Partners Plans lines of business unless noted below.

Gene therapy is a benefit exclusion for Individual and Family (ACA) product lines and therefore, non-covered.

POLICY STATEMENT

The plan considers Delandistrogene moxeparvovec-rokl (Elevidys®) medically necessary for its FDA approved indications when the prior authorization criteria listed in this policy are met.

FDA APPROVED INDICATIONS

Gene Therapy is the introduction, removal, or change in the content of a person's genetic code with the goal of treating or curing a disease. It includes therapies such as gene transfer, gene modified cell therapy, and gene editing.

Elevidys® is an adeno-associated virus vector-based gene therapy indicated for the treatment of patients 4 years and older with Duchenne muscular dystrophy (DMD) who are ambulatory and have a confirmed mutation in the DMD gene.

Limitation of Use:

Elevidys® is not recommended in patients with:

- Preexisting liver impairment (defined as gamma-glutamyl transferase [GGT] > 2 x upper limit of normal or total bilirubin > the upper limit of normal not due to Gilbert's syndrome) or

active hepatic viral infection due to the high risk of acute serious liver injury and acute liver failure.

- Recent vaccination (within 4 weeks of treatment) due to immunogenicity and potential safety concerns.

- Active or recent (within 4 weeks) infections due to safety concerns.

OFF-LABEL USE

N/A

PRIOR AUTHORIZATION CRITERIA

Prior authorization is required for Elevidys ® (Delandistrogene moxeparvovec-rokl).

Elevidys® (Delandistrogene moxeparvovec-rokl) may be considered medically necessary when **all** of the following apply:

1. Ambulatory members at least 4 years of age with a diagnosis of Duchenne Muscular Dystrophy (DMD) and confirmed mutation within the DMD gene.

    a. Must have proper documentation of DMD.

    b. Medication is being prescribed by, or in consultation with, a Neurologist, Neuromuscular specialist, or by a Muscular Dystrophy Association (MDA) clinic.

    c. FDA approved dosing.

    d. Single-dose intravenous use only.

    e. 1.33×10^{14} vector genomes (vg) per kg.

2. Able to receive premedication with corticosteroids 1 day prior to infusion and minimum of 60 days after (unless tapering is clinically indicated).

3. Does not have any deletion in exon 8 and/or exon 9 in the DMD gene.

4. Does not have any clinical signs or symptoms of active infection at the time of administration.

5. Individual is compliant with necessary monitoring parameters.

6. Individual has an anti-AAVrh74 total binding antibody titer <1:400.

7. Has received all age-appropriate vaccines based on current immunization schedules; vaccines should be administered ≥4 weeks prior to initiation of corticosteroid regimen.

8. Individual is not treated with any other RNA antisense agent or any other gene therapy. E.g., exon skipping therapies (Amondys 45™, Exondys 51®, Vyondys 53™).

RENEWAL CRITERIA

Re-administration of Elevidys is not recommended.

DOSAGE AND ADMINISTRATION

Elevidys® is a suspension for intravenous infusion with a nominal concentration of 1.33×10^{13} vg/mL, provided in a customized kit containing ten to seventy 10 mL single-dose vials, with each kit constituting a dosage unit based on the patient's body weight. Elevidys® is for single-dose intravenous infusion only.

1. **Recommended dosage:**
   a. **10 to 70 kg**: 1.33 x10^{14} vector genomes (vg) per kg of body weight.
   b. **>70 kg:** 9.31 x10^{15} vg
2. **Administration:**
   a. Instruct patient to maintain proximity to an appropriate healthcare facility, as determined by the healthcare provider, for at least 2 months following Elevidys® infusion.
   b. Select patients for treatment with Elevidys® with anti-AAVrh74 total binding antibody titers <1:400.
   c. Postpone in patients with active or recent (within 4 weeks) infections.
   d. Assess liver functions before infusion.
   e. Obtain platelet counts and troponin-I before infusion.
   f. One day **or** one week (depending on baseline corticosteroid regime), prior to infusion, initiate a corticosteroid regimen for a minimum of 60 days. Recommend modified corticosteroid dose for patients with liver function abnormalities.
   g. Administer as an intravenous infusion over 1-2 hours. Infuse at a rate of less than 10 mL/kg/hour**.**

RISK FACTORS/SIDE EFFECTS

Risk Factors:

1. **Serious Infections:** Serious infections with fatal outcomes may occur due to concomitant administration of corticosteroids, additional immunosuppressants, and ELEVIDYS. Monitor patients for signs and symptoms of infections and treat appropriately.

2. **Infusion-related Reactions:** Hypersensitivity reactions and anaphylaxis. Monitor during administration and for at least 3 hours after end of infusion. If symptoms occur, slow or stop infusion and give appropriate treatment. Once symptoms resolve, restart infusion at a slower infusion rate. Discontinue infusion for anaphylaxis.
3. **Acute Serious Liver Injury and Acute Liver Failure:** Acute serious liver injury in some cases fatal, has been observed. If acute serious liver injury is suspected, a consultation with a specialist is recommended. Patients with preexisting liver impairment, chronic hepatic condition or acute liver disease may be at higher risk of acute serious liver injury. Onset of liver injury typically begins 8 weeks after administration of Elevidys. Acute liver failure with fatal outcome has occurred in clinical and post-marketing settings in non-ambulatory patients.

4. **Immune-mediated Myositis:** Patients with deletions in the DMD gene in exons 1 to 17 and/or exons 59 to 71 may be at risk for severe immune-mediated myositis reaction. Consider additional immunomodulatory treatment if symptoms of myositis occur.
5. **Myocarditis**: Myocarditis and troponin-I elevations have been observed.
6. **Pre-existing Immunity against AAVrh74**: Perform baseline testing for presence of anti-AAVrh74 total binding antibodies prior to administration.

MONITORING

Monitoring:

1. The most common adverse reactions (incidence ≥ 5%) reported in clinical studies are vomiting, nausea, liver function test abnormalities, pyrexia, and thrombocytopenia.

2. Check liver function (clinical examination and laboratory testing including aspartate aminotransferase (AST), alanine aminotransferase (ALT), gamma-glutamyl transferase (GGT), albumin, and total bilirubin); activated partial thromboplastin time (aPTT), international normalized ratio (INR), and weekly for the first 3 months after infusion. Continue monitoring until results are unremarkable.

3. Check Troponin I before infusion, and weekly for the first month after infusion.

4. Check platelet counts weekly for the first two weeks.

CONTRAINDICATIONS

Contraindicated in patients with deletion of any portion or the entirety of exon 8 and/or exon 9, in the DMD gene.

BLACK BOX WARNING

Acute Serious Liver Injury and Acute Liver Failure: Prior to administration, assess liver function by clinical examination and laboratory testing. Administer systemic corticosteroids before and after ELEVIDYS infusion. Continue to monitor liver function weekly for the first 3 months after infusion and continue until results are unremarkable.

CLINICAL EVIDENCE

Delandistrogene moxeparvovec received accelerated approval in the United States by the FDA in June 2023 for the treatment of ambulatory boys with DMD, ages four through five years with a proven pathogenic variant in the DMD gene. The approval was expanded in June 2024 to traditional approval for ambulatory individuals four years of age and older with a confirmed pathogenic variant in the DMD gene.

FDA approval was based in part upon the surrogate outcome of increased microdystrophin production demonstrated in two small trials; microdystrophin protein expression measured by Western blot of muscle biopsies was significantly increased compared with baseline after 12 weeks of treatment. In one trial (n = 41), there was no improvement in functional outcome as measured by the mean change from baseline to week 48 in the North Star Ambulatory Assessment (NSAA), a 17-

item rating scale of functional motor abilities in ambulatory children with DMD .The mean change in NSAA score for the delandistrogene moxeparvovec group compared with the placebo group (1.7 versus 0.9) was not statistically significant. In a subgroup analysis, there was a greater improvement in the mean change from baseline in the NSAA score for children in the four- to five-year age group assigned to delandistrogene moxeparvovec compared with placebo (4.3 versus 1.9), while there was no difference between groups for children in the six- to seven-year age group (-0.2 versus 0.5).

A confirmatory randomized-controlled trial with 125 patients found a trend to improvement for functional outcome on the NSAA score for the delandistrogene moxeparvovec group compared with the placebo group (2.6 versus 1.9), but the difference did not reach statistical significance. However, delandistrogene moxeparvovec treatment led to benefit on several prespecified secondary endpoints, including the time to rise and 10-meter walk tests.

The effect of Elevidys micro-dystrophin expression was also evaluated in Study 2, an ongoing, open-label, multi-center study cohort consisting of 20 males DMD patients aged 4 through 7 years. All subjects received a single intravenous infusion of 1.33 × 1014 vg/kg.

For subjects aged 4 through 5 years who received 1.33 × 1014 vg/kg, the mean (SD) micro-dystrophin expression levels at Week 12 following infusion were 95.7% (N=3, SD: 17.9%) in Study 1 Parts 1 and 2 and 51.7% (N=11, SD: 41.0%) in Study 2 Cohort 1.

BACKGROUND

Delandistrogene moxeparvovec-rokl (Elevidys®) is a recombinant gene therapy product that is comprised of a non-replicating, recombinant, adeno-associated virus (AAV) serotype rh74 (AAVrh74) capsid and a ssDNA expression cassette flanked by inverted terminal repeats (ITRs) derived from AAV2. The cassette contains: 1) an MHCK7 gene regulatory component comprising a creatine kinase 7 promoter and an α-myosin heavy chain enhancer, and 2) the DNA transgene encoding the engineered ELEVIDYS micro-dystrophin protein. Elevidys® is designed to carry a transgene encoding a micro-dystrophin protein consisting of selected domains of dystrophin expressed in normal muscle cells. Micro-dystrophin has been demonstrated to localize to the sarcolemma. Following IV administration, the drug's vector genome undergoes distribution via systemic circulation and distributes into target muscle tissues followed by elimination in the urine and feces.

Its biodistribution and tissue transduction are detected in the target muscle tissue groups and quantified in the gastrocnemius or biceps femoris biopsies obtained from patients with mutations in the DMD gene. Evaluation of the drug's vector genome exposure in clinical muscle biopsies at Week 12 post-dose expressed as copies per nucleus revealed ELEVIDYS drug distribution and transduction with a mean change from baseline of 2.91 and 3.44 copies per nucleus at the recommended dose of 1.33×10^{14} vg/kg. Elevidys® is the first adeno-associated virus vector-based gene therapy indicated for the treatment of ambulatory members with DMD at least 4 years of age with Duchenne

muscular dystrophy (DMD) with a confirmed mutation in the DMD gene**.**

It is not curative and can be administered in an outpatient setting. Although it produces microdystrophin protein, the clinical benefit of delandistrogene moxeparvovec is not established.

CODING

Note*:* The Current Procedural Terminology (CPT®), Healthcare Common Procedure Coding System (HCPCS), and the 10th revision of the International Statistical Classification of Diseases and Related Health Problems (ICD-10) codes that *may* be listed in this policy are for reference purposes only. Listing of a code in this policy does not imply that the service is covered and is not a guarantee of payment. Other policies and coverage guidelines may apply. When reporting services, providers/facilities should code to the highest level of specificity using the code that was in effect on the date the service was rendered. This list may not be all inclusive.

CPT® is a registered trademark of the American Medical Association.

CPT Code	Description
N/A	N/A

HCPCS Code	Description
J1413	Injection, delandistrogene moxeparvovec – rokl, per therapeutic dose

ICD-10 Codes	Description
G71.01	Duchenne or Becker muscular dystrophy

DISCLAIMER

Approval or denial of payment does not constitute medical advice and is neither intended to guide nor influence medical decision making. Policy Bulletins are developed to assist in administering plan benefits and constitute neither offers of coverage nor medical advice.

This Policy Bulletin may be updated and therefore is subject to change. For Health Partners Plans Medicaid and Health Partners Plans Chip products: Any requests for services that do not meet criteria set in PARP will be evaluated on a case-by-case basis.

POLICY HISTORY

This section provides a high-level summary of changes to the policy since the previous version.

Summary	Version	Version Date
2026 Ad-hoc review. FDA prescribing information updated. Black box warning, Risk Factor/Side Effects, Dosage and Administration Sections updated. References updated.	C	10/16/2025
2025 Annual review. Revisions made to FDA indications, Prior Authorization Criteria, Renewal Criteria, Risk Factor/Side Effects, Clinical Evidence, Dosage and Administration Sections. ICD 10 codes added. References updated.	C	10/16/2025
FDA age indication updated.	B	11/20/2024
New policy.	A	07/01/2024

REFERENCES

1. Elevidys (delandistrogene moxeparvovec) [prescribing information]. Cambridge, MA: Sarepta Therapeutics Inc; November 2025. Accessed November 19, 2025. Available at https://www.elevidys.com/pi

2. National Library of Medicine clinical trials.gov: https://classic.clinicaltrials.gov/ct2/show/NCT03375164?term=Delandistrogene+moxeparvovec&dra w=2&rank=4

3. Duchenne and Becker muscular dystrophy: Glucocorticoid and disease-modifying treatment-Basil T Darras, MD, Up To Date, last updated: **Sep 15, 2025**