Clinical Policy: Delandistrogene moxeparvovec-rokl (Elevidys)

Reference Number: PA.CP.PHAR.593
Effective Date: 08/2023
Last Review Date: 07/2023

Description
Delandistrogene moxeparvovec-rokl (Elevidys) is an adeno-associated virus vector-based gene therapy.

FDA Approved Indication(s)
Elevidys is 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.

This indication is approved under accelerated approval based on expression of Elevidys micro-dystrophin in skeletal muscle observed in patients treated with Elevidys. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial(s).

Policy/Criteria
Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.

It is the policy of PA Health & Wellness® that Elevidys is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

A. Duchenne Muscular Dystrophy (must meet all)**:**

1. Diagnosis of DMD confirmed by genetic testing;
2. Member does not have a deletion in exon 8 and/or 9 in the DMD gene;
3. One of the following (a or b):
    a. Prescribed by or in consultation with a neurologist;
    b. Member is being treated at a certified Duchenne care center or an MDA care center (see Appendix D);
4. Age ≥ 4 years and ≤ 5 years;
5. Member has ambulatory function
6. Documentation of baseline laboratory tests demonstrating anti-AAVrh74 total binding antibody titers < 1:400 as determined by ELISA binding immunoassay;
7. Elevidys is prescribed concurrently with an oral corticosteroid, unless contraindicated or clinically significant adverse effects are experienced;
8. Member has not been previously treated with Elevidys;
9. Elevidys is not prescribed concurrently with exon skipping therapies (e.g., Amondys 45™, Exondys 51®, Viltepso™, Vyondys 53™);
10. If member is currently on exon skipping therapy (e.g., Amondys 45, Exondys 51, Viltepso, Vyondys 53), both of the following (a and b):

a. Provider must submit evidence of clinical deterioration (e.g., significant decline in 6MWT, LVEF, or FVC over a period of 3 to 6 months) while on exon skipping therapy;
b. Documentation of provider attestation of clinical deterioration and discontinuation of exon skipping therapy;

11. Dose does not exceed 1.33 x 10^{14} vector genomes (vg) per kg.

Approval duration: 3 months (one time infusion per lifetime)

B. Other diagnoses/indications

1. Refer to the off-label use policy if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): PA.CP.PMN.53

II. Continued Therapy

A. Diagnosis (must meet all)**:**

1. Continued therapy will not be authorized as Elevidys is indicated to be dosed one time only.

Approval duration: Not applicable

B. Other diagnoses/indications (must meet 1 or 2)**:**

1. Currently receiving medication via PA Health & Wellness benefit and documentation supports positive response to therapy or the Continuity of Care policy (PA.LTSS.PHAR.01) applies.
   Approval duration: Duration of request or 12 months (whichever is less); or
2. Refer to the off-label use policy for the relevant line of business if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): PA.CP.PMN.53

III. Diagnoses/Indications for which coverage is NOT authorized:

A. Non-FDA approved indications, which are not addressed in this policy, unless there is sufficient documentation of efficacy and safety according to the off label use policies – PA.CP.PMN.53

IV. Appendices/General Information

Appendix A: Abbreviation/Acronym Key

6MWT: 6-minute walk test
AAN: American Academy of Neurology
DMD: Duchenne muscular dystrophy
FDA: Food and Drug Administration
FVC: forced vital capacity
LVEF: left ventricular ejection fraction
MDA: muscular dystrophy association
PPMD: parent project muscular dystrophy
vg: vector genomes

Appendix B: Therapeutic Alternatives

Not Applicable

Appendix C: Contraindications/Boxed Warnings

- Contraindication(s): patients with any deletion in exon 8 and/or exon 9 in the *DMD gene*
- Boxed warning(s): none

Appendix D: General Information

- Corticosteroids are routinely used in DMD management with established efficacy in slowing decline of muscle strength and function (including motor, respiratory, and cardiac). They are recommended for all DMD patients per the American Academy of Neurology (AAN) and DMD Care Considerations Working Group; in addition, the AAN guidelines have been endorsed by the American Academy of Pediatrics, the American Association of Neuromuscular & Electrodiagnostic Medicine, and the Child Neurology Society.
  - The DMD Care Considerations Working Group guidelines, which were updated in 2018, continue to recommend corticosteroids as the mainstay of therapy.
- Prednisone is the corticosteroid with the most available evidence. A second corticosteroid commonly used is Emflaza (deflazacort), which was FDA approved for DMD in February 2017.
- Parent Project Muscular Dystrophy (PPMD)'s certified Duchenne care center program helps to ensure that centers comply with the standards of care and services established in the Duchenne care guidelines. The full list of certified Duchenne care centers can be found at: https://www.parentprojectmd.org/care/find-a-certified-duchenne-care-center/.
- The Muscular Dystrophy Association (MDA) care centers offers individuals with muscular dystrophy, ALS and other neuromuscular diseases to access expert multidisciplinary care, clinical trials, and to connect with MDA and the neuromuscular community. The full list of MDA care centers can be found at: https://www.mda.org/care/care-center-list.

V. Dosage and Administration

Indication	Dosing Regimen	Maximum Dose
DMD	1.33×10^{14} vg/kg body weight as a single-dose IV infusion	1.33×10^{14} vg/kg body weight

VI. Product Availability

Customized kit containing ten to seventy 10 mL single-dose vials, constituting a dosage unit based on the patient's body weight

VII. References

1. Elevidys Prescribing Information. Cambridge, MA: Sarepta Therapeutics, Inc.; June 2023. Available at: https://www.elevidys.com/PI. Accessed June 23, 2023.
2. ClinicalTrials.gov. A randomized, double-blind, placebo-controlled study of SRP-9001 (delandistrogene moxeparvovec) for Duchenne muscular dystrophy (DMD). Available at: https://www.clinicaltrials.gov/ct2/show/NCT03769116. Accessed June 23, 2023.
3. ClinicalTrials.gov. A gene transfer therapy study to evaluate the safety of and expression from SRP-9001 (delandistrogene moxeparvovec) in participants with Duchenne muscular dystrophy (DMD) (ENDEAVOR). Available at: https://www.clinicaltrials.gov/ct2/show/NCT04626674. Accessed June 23, 2023.
4. Gloss D, Moxley RT, Ashwal S, Oskoui M. 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. Reaffirmed January 22, 2022.

5. Birnkrant DJ, Bushby K, Bann CM, et al. Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and neuromuscular, rehabilitation, endocrine, and gastrointestinal and nutritional management. Lancet Neurol. 2018; 17(3):251-267.

Coding Implications
Codes referenced in this clinical policy are for informational purposes only. Inclusion or exclusion of any codes does not guarantee coverage. Providers should reference the most up-to-date sources of professional coding guidance prior to the submission of claims for reimbursement of covered services.

HCPCS Codes	Description
J3590	Unclassified biologics
C9399	Unclassified drugs or biologicals

Reviews, Revisions, and Approvals	Date	P&T Approval Date
Policy created	07/2023	