

**Clinical Policy: Delandistrogene moxeparvovec-rokl (Elevidys)**

Reference Number: PA.CP.PHAR.593

Effective Date: 08/2023

Last Review Date: 07/2025

**Description**

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

**FDA Approved Indication(s)**

Elevidys is indicated in individuals at least 4 years of age:

- For the treatment of Duchenne muscular dystrophy (DMD) in patients who are ambulatory and have a confirmed mutation in the *DMD* gene.
- For the treatment of DMD in patients who are non-ambulatory and have a confirmed mutation in the *DMD* gene.\*

*\*This indication is approved under accelerated approval based on expression of Elevidys micro-dystrophin (noted thereafter as “micro-dystrophin”). 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  $\geq$  4 years
5. Documentation of baseline laboratory tests demonstrating anti-AAVrh74 total binding antibody titers  $< 1:400$  as determined by ELISA binding immunoassay;
6. Elevidys is prescribed concurrently with an oral corticosteroid, unless contraindicated or clinically significant adverse effects are experienced;
7. Member has not been previously treated with Elevidys;
8. Elevidys is not prescribed concurrently with exon skipping therapies (e.g., Amondys 45™, Exondys 51®, Vilepso™, Vyondys 53™);
9. If member is currently on exon skipping therapy (e.g., Amondys 45, Exondys 51, Vilepso, 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;
10. Dose does not exceed  $1.33 \times 10^{14}$  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. Duchenne Muscular Dystrophy (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.PHARM.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 x 10 <sup>14</sup> vg/kg body weight as a single-dose IV infusion | 1.33 x 10 <sup>14</sup> 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.; August 2024. Available at: <https://www.elevidys.com/PI>. Accessed May 22, 2025.
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 May 22, 2025.
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 May 22, 2025.
4. SRP-9001 (delandistrogene moxeparvovec) for the treatment of Duchenne muscular dystrophy (DMD). Sponsor briefing document prepared for the Cellular, Tissue, and Gene Therapies Advisory Committee meeting May 12, 2023. Available at: <https://www.fda.gov/media/168022/download>. Accessed May 22, 2025.

5. 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.
6. 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.
7. Coratti G, Pane M, Brogna C, et al. Gain and loss of upper limb abilities in Duchenne muscular dystrophy patients: A 24-month study. *Neuromuscular Disorders*. 2024;34:75-82.
8. FDA.gov. FDA news release - FDA expands approval of gene therapy for patients with Duchenne muscular dystrophy. June 20, 2024. Available at: <https://www.fda.gov/news-events/press-announcements/fda-expands-approval-gene-therapy-patients-duchenne-muscular-dystrophy>. Accessed May 22, 2025.
9. FDA.gov. Review Memo – Integrated clinical and clinical pharmacology. June 18, 2024. Available at: <https://www.fda.gov/vaccines-blood-biologics/tissue-tissue-products/elevidys>. Accessed May 22, 2025.
10. Oskoui M, Caller TA, Parsons JA, et al. Delandistrogene moxeparvovec gene therapy in individuals with Duchenne muscular dystrophy: Evidence in focus. Report of the AAN guidelines subcommittee. *Neurology* 2025;104:e213604.

### 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                                                        |
|-------------|--------------------------------------------------------------------|
| J1413       | Injection, delandistrogene moxeparvovec-rokl, per therapeutic dose |

| Reviews, Revisions, and Approvals                                                                                                                      | Date    |
|--------------------------------------------------------------------------------------------------------------------------------------------------------|---------|
| Policy created                                                                                                                                         | 07/2023 |
| 3Q 2024 annual review: removed under 5 years old and has ambulatory function from criteria; added HCPCS code [J1413]; references reviewed and updated. | 07/2024 |
| 3Q 2025 annual review: no significant changes; references reviewed and updated.                                                                        | 07/2025 |