# Clinical Policy: Delandistrogene Moxeparvovec-rokl (Elevidys)

Reference Number: CP.PCH.56
Effective Date: 06.01.25
Last Review Date: 08.25
Line of Business: Commercial, HIM

Coding Implications
Revision Log

**See Important Reminder at the end of this policy for important regulatory and legal information.**

## 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.*

All requests reviewed under this policy **require Precision Drug Action Committee (PDAC) Utilization Management Review**. Refer to CC.PHAR.21 for process details.

It is the policy of health plans affiliated with Centene Corporation® that Elevidys is **not medically necessary** for its FDA-approved indication:

**I. Elevidys is considered experimental and investigational and not medically necessary for the treatment of DMD patients at least 4 years of age who are ambulatory or non-ambulatory for the following reasons:**

**A. Elevidys does not have proven efficacy in the treatment of DMD for ambulatory patients.**

1. The phase III EMBARK [NCT05096221] confirmatory trial, which evaluated patients aged 4 to 7 years, failed to meet its statistical primary endpoint of improvement versus placebo in the North Star Ambulatory Assessment (NSAA) total score.
2. Study 102 Part 1 [NCT03769116], which evaluated patients aged 4 years to 7 years, failed to demonstrate a statistically significant change in NSAA from baseline to week 48 after treatment. Data showed no clear association between expression of Elevidys micro-dystrophin and change in NSAA total score.

**B. Elevidys does not have proven efficacy in the treatment of DMD for non-ambulatory patients.**

1. The phase I ENDEAVOR - Study 103 [NCT04626674] was the only study that contained data on non-ambulatory patients with DMD. Study 103 was not designed to demonstrate clinical efficacy and there was no data to support effectiveness for non-ambulatory patients with DMD.

## II. Appendices/General Information

*Appendix A: Abbreviation/Acronym Key*
DMD: Duchenne muscular dystrophy
FDA: Food and Drug Administration
NSAA: North Star Ambulatory Assessment

*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: Expanded Elevidys Indication*

- In making the decision to expand the Elevidys indication, the FDA considered the totality of the evidence from two double-blind, placebo-controlled studies (Study 1 [NCT03769116] and Study 3 [EMBARK; NCT05096221]) and one open-label study (Study 2 [ENDEAVOR; NCT04626674]).
  - For the traditional approval for ambulatory patients aged ≥ 4 years, while the large, randomized EMBARK trial failed to meet its statistical primary endpoint of improvement versus placebo in the North Star Ambulatory Assessment (NSAA), the FDA found the observations regarding the secondary endpoints and exploratory endpoints to be compelling and to indicate clinical benefit compared to placebo. These endpoints included improvements in time to rise from the floor, 10-meter walk/run, time to ascend four steps, and creatine kinase levels.
  - In granting accelerated approval for non-ambulatory individuals aged ≥ 4 years, the FDA extrapolated clinical data from ambulatory individuals indicating a correlation of Elevidys micro-dystrophin levels with clinical outcome measures. Based on the evidence and given that the mechanism of action of Elevidys is similar for ambulatory and non-ambulatory populations, the FDA determined that an increased level of micro-dystrophin is reasonably likely to predict clinical benefit in the non-ambulatory population.

## III. Dosage and Administration

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

## IV. Product Availability

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

## V. 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. ClinicalTrials.gov. A gene transfer study to evaluate the safety and efficacy of delandistrogene moxeparvovec (SRP-9001) in participants with Duchenne muscular dystrophy (DMD)(EMBARK). Available at: https://www.clinicaltrials.gov/study/NCT05096221. 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 | P&T Approval Date |
|---|---|---|
| Policy created (adopted from CP.PHAR.593). | 03.27.25 | 05.25 |
| 3Q 2025 annual review: no significant changes; references reviewed and updated. | 05.22.25 | 08.25 |
| Updated language under Policy/Criteria to effectively redirect prior authorization reviews to Precision Drug Action Committee (PDAC) Utilization Management Review. | 11.03.25 | |

**Important Reminder**

This clinical policy has been developed by appropriately experienced and licensed health care professionals based on a review and consideration of currently available generally accepted standards of medical practice; peer-reviewed medical literature; government agency/program approval status; evidence-based guidelines and positions of leading national health professional organizations; views of physicians practicing in relevant clinical areas affected by this clinical policy; and other available clinical information. The Health Plan makes no representations and accepts no liability with respect to the content of any external information used or relied upon in developing this clinical policy. This clinical policy is consistent with standards of medical practice current at the time that this clinical policy was approved. "Health Plan" means a health plan that has adopted this clinical policy and that is operated or administered, in whole or in part, by Centene Management Company, LLC, or any of such health plan's affiliates, as applicable.

The purpose of this clinical policy is to provide a guide to medical necessity, which is a component of the guidelines used to assist in making coverage decisions and administering benefits. It does not constitute a contract or guarantee regarding payment or results. Coverage decisions and the administration of benefits are subject to all terms, conditions, exclusions, and limitations of the coverage documents (e.g., evidence of coverage, certificate of coverage, policy, contract of insurance, etc.), as well as to state and federal requirements and applicable Health Plan-level administrative policies and procedures.

This clinical policy is effective as of the date determined by the Health Plan. The date of posting may not be the effective date of this clinical policy. This clinical policy may be subject to applicable legal and regulatory requirements relating to provider notification. If there is a discrepancy between the effective date of this clinical policy and any applicable legal or regulatory requirement, the requirements of law and regulation shall govern. The Health Plan retains the right to change, amend or withdraw this clinical policy, and additional clinical policies may be developed and adopted as needed, at any time.

This clinical policy does not constitute medical advice, medical treatment, or medical care. It is not intended to dictate to providers how to practice medicine. Providers are expected to exercise professional medical judgment in providing the most appropriate care, and are solely responsible for the medical advice and treatment of members. This clinical policy is not intended to recommend treatment for members. Members should consult with their treating physician in connection with diagnosis and treatment decisions.

Providers referred to in this clinical policy are independent contractors who exercise independent judgment and over whom the Health Plan has no control or right of control. Providers are not agents or employees of the Health Plan.