

Clinical Policy: Delandistrogene Moxeparvovec-rokl (Elevidys)

Reference Number: CP.PCH.56

Effective Date: 06.01.25

Last Review Date: 08.26

Line of Business: Commercial, HIM/ICHRA

[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 for the treatment of patients 4 years of age and older with Duchenne muscular dystrophy (DMD) who are ambulatory and have a confirmed mutation in the *DMD* gene.

Limitations 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

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): acute serious liver injury and acute liver failure

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 [EMBARC; 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.
- On November 14, 2025, the FDA approves new safety warning and revised indication that limits use for Elevidys following reports of fatal liver injury, including:

- Addition of a Boxed Warning describing the risk of serious liver injury and acute liver failure, including fatal outcomes;
- Limiting the indication to ambulatory patients with DMD who are 4 years of age and older with a confirmed mutation in the DMD gene;
- Removal of the indication for non-ambulatory patients with DMD;
- Addition of a Limitations of Use statement to guide clinical decision-making.

III. Dosage and Administration

Indication	Dosing Regimen	Maximum Dose
DMD	1.33 x 10 ¹⁴ vg/kg body weight as a single-dose IV infusion	1.33 x 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.; November 2025. Available at: <https://www.elevidys.com/PI>. Accessed April 9, 2026.
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 28, 2026.
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 28, 2026.
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 28, 2026.
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 28, 2026.
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 28, 2026.

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.
11. FDA.gov. FDA news release – FDA approves new safety warning and revised indication that limits use for Elevidys following reports of fatal liver. November 14, 2025. Available at: <https://www.fda.gov/news-events/press-announcements/fda-approves-new-safety-warning-and-revised-indication-limits-use-elevidys-following-reports-fatal>. Accessed May 28, 2026.

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	
3Q 2026 annual review: updated FDA approved indication with removal of non-ambulatory indication per updated PI; added ICHRA line of business; references reviewed and updated.	04.09.26	08.26

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.

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