Clinical Policy: Delandistrogene Moxeparvovec-rokl (Elevidys)

Reference Number: CP.PHAR.593
Effective Date: 06.22.23
Last Review Date: 08.25
Line of Business: Medicaid

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 **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

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

**Only for initial treatment dose; subsequent doses will not be covered.*

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 < 6 years*;
   ***A state-mandated age limit exists for FL Medicaid; approvals beyond this age range will not be considered for medical necessity.*
   AHCA Summary of Drug Limitations, page 28, 1/31/2025

5. Member is ambulatory* (i.e., able to walk without assistive devices, not wheelchair dependent);
   *For non-ambulatory members, please refer to section III.
6. Member does not have an active infection;
7. Member has all of the following assessed within the last 30 days (a, b, c, and d):
   a. Stable cardiac function with left ventricular ejection fraction (LVEF) ≥ 40%;
   b. Baseline liver function tests (e.g., AST, ALT, bilirubin) with absence of significant liver dysfunction, defined as one of the following: acute liver disease, pre-existing liver dysfunction, chronic hepatic condition;
   c. Baseline platelet count;
   d. Baseline troponin I;
8. Documentation of baseline laboratory tests demonstrating anti-AAVrh74 total binding antibody titers < 1:400 as determined by ELISA binding immunoassay;
9. One of the following (a or b):
   a. Member has been on a stable dose of an oral corticosteroid (e.g., prednisone, Emflaza®*, Agamree®*) for ≥ 3 months, unless contraindicated or clinically significant adverse effects are experienced;
      **Prior authorization is required for Emflaza and Agamree*
   b. Provider attestation that member will be initiated on standard of care oral corticosteroid prior to and following Elevidys;
10. Elevidys is prescribed concurrently with a prophylactic corticosteroid regimen, unless contraindicated or clinically significant adverse effects are experienced;
11. Elevidys is not prescribed concurrently with exon skipping therapies (e.g., Amondys 45™, Exondys 51®, Viltepso™, Vyondys 53™);
12. If member is currently on exon skipping therapy (e.g., Amondys 45, Exondys 51, Viltepso, Vyondys 53), member must discontinue therapy prior to Elevidys and not-reinitiate exon skipping therapy after Elevidys;
13. Member has not been previously treated with Elevidys;
14. Member has not been previously treated with the investigational agent deramiocel (CAP-1002);
15. Current documentation (within the last 90 days) of member's body weight (in kg);
16. Dose does not exceed 1.33×10^{14} vector genomes (vg) per kg.

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

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

1. If this drug has recently (within the last 6 months) undergone a label change (e.g., newly approved indication, age expansion, new dosing regimen) that is not yet reflected in this policy, refer to one of the following policies (a or b):
   a. For drugs on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the no coverage criteria policy for the relevant line of business: CP.PMN.255 for Medicaid; or
   b. For drugs NOT on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the non-formulary policy for the relevant line of business: CP.PMN.16 for Medicaid; or

2. If the requested use (e.g., diagnosis, age, dosing regimen) is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized) AND criterion 1 above does not apply, refer to the off-label use policy for the relevant line of business: CP.PMN.53 for Medicaid.

II. Continued Therapy

A. Duchenne Muscular Dystrophy

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. If this drug has recently (within the last 6 months) undergone a label change (e.g., newly approved indication, age expansion, new dosing regimen) that is not yet reflected in this policy, refer to one of the following policies (a or b):
   a. For drugs on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the no coverage criteria policy for the relevant line of business: CP.PMN.255 for Medicaid; or
   b. For drugs NOT on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the non-formulary policy for the relevant line of business: CP.PMN.16 for Medicaid; or
2. If the requested use (e.g., diagnosis, age, dosing regimen) is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized) AND criterion 2 above does not apply, refer to the off-label use policy for the relevant line of business: CP.PMN.53 for Medicaid.

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 – CP.PMN.53 for Medicaid, or evidence of coverage documents;

B. Elevidys for non-ambulatory DMD patients *(see Appendix D)* is considered experimental and investigational, and not a covered benefit. Please refer to evidence of coverage (EOC) documents;

C. Elevidys for ambulatory DMD outside the 4 to 5 years age group (*see Appendix D)* is considered experimental and investigational, and not a covered benefit. Please refer to evidence of coverage (EOC) documents.

IV. Appendices/General Information

Appendix A: Abbreviation/Acronym Key

AAN: American Academy of Neurology
DMD: Duchenne muscular dystrophy
FDA: Food and Drug Administration
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. On October 2023, a third corticosteroid, Agamree (vamorolone), was approved by the FDA for DMD.
- 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.
- Concurrent prophylactic corticosteroid regimen with Elevidys infusion
  - Immune responses to the AAVrh74 vector can occur after administration of Elevidys. To reduce the risk associated with an immune response, corticosteroids should be administered starting 1 day prior to Elevidys infusion. This regimen is recommended for a minimum of 60 days after the infusion, unless earlier tapering is indicated.
- Non-ambulatory status is defined as inability to walk independently for 10 meters or more.
- 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.

- At this time, the current evidence does not support medical necessity of Elevidys for the treatment of DMD in the following patients:
  1) Ambulatory patients outside the 4 to 5 years age group for the following reasons:
    - The phase III EMBARK confirmatory trial, which evaluated patients aged 4 to 7 years, failed to meet its statistical primary endpoint of improvement versus placebo in the NSAA total score.
    - Study 102 Part 1, 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. However, in an exploratory subgroup analysis data demonstrated that there was a numerical advantage for Elevidys in the NSAA total score for individuals in the age 4-5 years cohort, which was not found in the age 6 through 7 years cohort.
  2) Non-ambulatory patients for the following reasons:
    - Elevidys does not have proven efficacy in the treatment of DMD in patients who are non-ambulatory.
      - 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.
    - Elevidys is a one-time, single-dose infusion that is not amenable to treatment modification based on therapeutic response with extrapolation of data.

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.; 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 pre-emptively	09.27.22	11.22
RT1: Elevidys is now FDA approved – criteria updated per FDA labeling: revised age to 4 through 5 years; revised covered *DMD* gene mutations to exclude any deletion in exon 8 and/or 9; revised maximum dosing; add bypass of neurologist requirement if member is being treated at either a certified Duchenne care center	07.18.23	08.23

Reviews, Revisions, and Approvals	Date	P&T Approval Date
or an MDA care center; removed inadequate response to oral corticosteroids requirement and revised to "member has been on a stable dose of oral corticosteroid for ≥ 3 months" per clinical studies; added disclaimer under Policy/Criteria "All requests reviewed under this policy **require medical director review;"** references reviewed and updated.		
For members currently on exon skipping therapies, removed requirement of "significant decline while on exon skipping therapies" and revised to "member must discontinue exon skipping therapy prior to Elevidys and not-reinitiate exon skipping therapy after Elevidys"; added bypass of stable dose of corticosteroids with option for provider attestation that member will be initiated on standard of care oral corticosteroid prior to and following Elevidys; for concurrent corticosteroid clarified that it is a "prophylactic" corticosteroid regimen. Added HCPCS code [J1413].	08.25.23	11.23
For ambulatory status, clarified as "ability to walk without assistive devices, not wheelchair dependent"; for functional assessments removed 6MWT distance ≥ 200 m and added the following: NSAA, 100-meter timed test, time to ascend 4 steps, time to rise from the floor and 10-meter timed test based on Study 102 age group 4-5 years baseline characteristics.	11.27.23	12.23
Removed the following functional assessments: NSAA, 100-meter timed test, time to ascend 4 steps, time to rise from the floor, and 10-meter timed test.	02.13.24	05.24
3Q 2024 annual review: no significant changes; references reviewed and updated. RT4: updated FDA approved indications with conversion from accelerated approval to full approval for patients with DMD ≥ 4 years of age and ambulatory plus expansion to non-ambulatory patients (accelerated approval); revised age restrictions to ≥ 4 years (previously ≥ 4 and ≤ 5 years); added treatment of non-ambulatory DMD patients to Section III Diagnosis/Indications not covered based on current available evidence.	07.16.24	08.24
Added requirement for current documentation (within the last 90 days) of member's body weight (in kg).	12.26.24	
Removed HIM and Commercial lines of business with disclaimer to refer to Section III; restricted Elevidys to age to 4 years through 5 years; added member does not have an active infection; added member has all of the following assessed within the last 30 days: stable cardiac function with LVEF ≥ 40%, baseline liver function tests with absence of significant liver dysfunction, and baseline platelet count and baseline troponin I; added disclaimer for non-ambulatory members to refer to Section III; added member has not	03.11.25	03.25 (adhoc)

Reviews, Revisions, and Approvals	Date	P&T Approval Date
been previously treated with the investigational agent deramiocel (CAP-1002).		
Removed HIM and Commercial line of business (refer to CP.PCH.56); clarified age restriction to age to 4 years and < 6 years.	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.	11.04.25	

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.

This clinical policy is the property of the Health Plan. Unauthorized copying, use, and distribution of this clinical policy or any information contained herein are strictly prohibited. Providers, members, and their representatives are bound to the terms and conditions expressed herein through the terms of their contracts. Where no such contract exists, providers, members and their representatives agree to be bound by such terms and conditions by providing services to members and/or submitting claims for payment for such services.

Note:
For Medicaid members, when state Medicaid coverage provisions conflict with the coverage provisions in this clinical policy, state Medicaid coverage provisions take precedence. Please refer to the state Medicaid manual for any coverage provisions pertaining to this clinical policy.