Clinical Policy: Delandistrogene Moxeparvovec-rokl (Elevidys)

Reference Number: CP.PHAR.593
Effective Date: 06.22.23
Last Review Date: 12.23
Line of Business: Commercial, HIM, 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 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.

All requests reviewed under this policy **require medical director review.**

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 ≤ 5 years;
5. Member is ambulatory (e.g., able to walk without assistive devices, not wheelchair dependent);
6. Member's ambulatory function within the last 30 days meets one of the following (a, b, c, d, or e):
   a. North Star Ambulatory Assessment (NSAA) total score ≥ 15;

b. 100-meter timed test ≤ 70.1 seconds;
c. Time to ascend 4 steps ≤ 5.8 seconds;
d. Time to rise from the floor ≤ 5.2 seconds;
e. 10-meter timed test ≤ 7.2 seconds;

7. Documentation of baseline laboratory tests demonstrating anti-AAVrh74 total binding antibody titers < 1:400 as determined by ELISA binding immunoassay;
8. 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;
9. Elevidys is prescribed concurrently with a prophylactic corticosteroid regimen, unless contraindicated or clinically significant adverse effects are experienced;
10. Member has not been previously treated with Elevidys;
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. 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.CPA.190 for commercial, HIM.PA.33 for health insurance marketplace, and 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.CPA.190 for commercial, HIM.PA.103 for health insurance marketplace, and 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.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace, and 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.CPA.190 for commercial, HIM.PA.33 for health insurance marketplace, and 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.CPA.190 for commercial, HIM.PA.103 for health insurance marketplace, and 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.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace, and 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.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace, and CP.PMN.53 for Medicaid, or evidence of coverage 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
NSAA: North Star Ambulatory Assessment
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.

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. 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 November 27, 2023.
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.

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 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.	07.18.23	08.23
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:	11.27.23	12.23

Reviews, Revisions, and Approvals	Date	P&T Approval Date
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.		

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.

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.