Harvard Pilgrim Health Care | TUFTS Health Plan

Medical Necessity Guidelines

Medical Benefit Drug

Elevidys® (delandistrogene moxeparvovec-rokl)

Effective: July 1, 2026

Prior Authorization Required If REQUIRED, submit supporting clinical documentation pertinent to service request.	Yes ☒ No ☐

Applies to:

Commercial Products

☒ Harvard Pilgrim Health Care Commercial products; Fax 617-673-0988
☒ Tufts Health Plan Commercial products; Fax 617-673-0988

Public Plans Products

☒ Tufts Health Direct – A Massachusetts Qualified Health Plan (QHP) (a commercial product); Fax 617-673-0988
☐ Tufts Health Together – MassHealth Accountable Care Partnership Plans; Fax 617-673-0939
☒ Tufts Health RITogether – A Rhode Island Medicaid Plan; Fax 617-673-0939
☐ Tufts Health One Care-- A dual-eligible product; Fax 617-673-0956

Senior Products

☐ Tufts Health Plan Senior Care Options (SCO), (a dual-eligible product); Fax 617-673-0956
☐ Tufts Medicare Preferred HMO, (a Medicare Advantage product); Fax 617-673-0956
☐ Tufts Medicare Preferred PPO, (a Medicare Advantage product); Fax 617-673-0956

Note: While you may not be the provider responsible for obtaining prior authorization, as a condition of payment you will need to ensure that prior authorization has been obtained.

Overview

Duchenne muscular dystrophy (DMD) is a rare, life-limiting, progressive childhood disease that affects 1 in 3,500 – 5,000 live male births. It is an X-linked, degenerative neuromuscular disease caused by pathogenic variants in the DMD gene resulting in reduced or near absence of dystrophin, a protein that helps keep muscle cells intact. The population is almost exclusively male, with an average age at diagnosis of three to five years. DMD is characterized by progressive muscle weakness and wasting due to the absence of dystrophin protein that causes degeneration of skeletal and cardiac muscle results in disabling muscle weakness.

Elevidys (delandistrogene moxeparvovec-rokl) is a prescription adeno-associated virus vector-based gene therapy used to treat children beginning at age 4 with Duchenne muscular dystrophy (DMD) who have a confirmed pathogenic variant in the dystrophin gene. It utilizes an adeno-associated virus (AAVrh74) to introduce a shorter version of the DMD gene into muscle tissue, partially compensating for the lack of a functional DMD gene and targeting the underlying genetic defect that causes DMD. Elevidys was FDA approved under accelerated approval based on expression of Elevidys micro-dystrophin in skeletal muscle observed in patients treated with Elevidys. Elevidys is contraindicated in patients with any deletion in exon 8 and/or exon 9 in the DMD gene.

There are currently four new drugs that have been approved by the FDA to treat DMD: Amondys45 (casimersen), Exondys51 (eteplirsen), Viltepso (viltolarsen), and Vyondys53 (golodirsen) which are “exon-skipping drugs that allows the body to produce more of the dystrophin protein. In contrast, Elevidys is a different type of treatment and first of its kind, which works by addressing the underlying genetic defect that causes DMD.

Food and Drug Administration (FDA) Approved Indications:

- ELEVIDYS is an adeno-associated virus vector-based gene therapy indicated for the treatment of pediatric patients beginning at age 4 with Duchenne muscular dystrophy (DMD) with a confirmed pathogenic variant 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 additional trial(s).
- **NOTE**: In November 2025, the Elevidys prescribing information was updated to reflect **a black box safety warning**. A new

warning states that both acute serious liver injury and acute liver failure has occurred in patients who were given Elevidys. Patients should obtain prompt consultation with a specialist (e.g., gastroenterologist or hepatologist) if acute serious liver injury or impending acute liver failure is suspected.

- **Point32Health requires that Providers comply fully with the FDA-approved prescribing information unless the clinical guideline criteria listed below states otherwise. This includes adherence to all labeled indications, limitations of use, dosing parameters, administration requirements, monitoring during and after treatment, contraindications, safety precautions, and patient counseling, as outlined in the official FDA labeling.**

Clinical Guideline Coverage Criteria

The Plan may cover a **one-time-only dose** of Elevidys for treatment of muscular dystrophy when **All** the following criteria are met:

1. Documentation of Duchenne muscular dystrophy (DMD) diagnosis with a mutation in the DMD gene confirmed by genetic testing; **and**
2. Member is **4 or 5** years of age; **and**
3. Prescribed by, or in consultation with, a pediatric neurologist/neuromuscular specialist with expertise in the diagnosis of DMD; **and**
4. Documentation the Member does not have any deletion (partial or entire) in exon 8 and/or exon 9 in the DMD gene; **and**
5. Member will not receive concomitant therapy for DMD with antisense oligonucleotide therapy [e.g., Amondys45 (casimersen), Exondys51 (eteplirsen), Viltepso (viltolarsen), Vyondys53 (golodirsen)]; **and**
6. Member has not previously been treated with Elevidys; **and**
7. Member has a baseline measurement for **Both** of the following:
    a. Baseline North Star Ambulatory Assessment, including scores and times on individual items (within the past 3 months); **and**
    b. Baseline six-minute walk test (within the past 3 months); **and**
8. Documentation that the Member is **ambulatory** and does not require an assistive device (e.g., cane, walker, wheelchair, etc.); **and**
9. Provider attests and submits documentation confirming that there is no preexisting or current hepatic impairment as a result of viral infection, chronic disease, and platelet counts and troponin levels have been checked prior to infusion; **and**
10. Provider attests that platelet count, liver function, and troponin levels will be monitored following Elevidys administration as indicated in the FDA prescribing information; **and**
11. Provider attests that cardiac function has been addressed and LVEF is greater than 40%; **and**
12. Pre-treatment testing confirms baseline anti-AAVrh74 total binding antibody titers <1:400 using a Total Binding Antibody enzyme-linked immunosorbent assay (ELISA); **and**
13. Provider attests that the Member has no active or recent (within 4 weeks) infection; **and**
14. Member has been prescribed a stable dose of corticosteroids, unless contraindicated or intolerance, prior to start of therapy and corticosteroids will be used pre-and post- infusion according to label directions; **and**
15. Provider attests that the Member's vaccinations were completed at least 4 weeks prior to initiation of corticosteroid regimen due to immunogenicity and potential safety concerns; **and**
16. Infusion will take place at a qualified treatment facility with immediate availability for treatment of infusion-related reactions; **and**
17. The Member has been instructed to maintain proximity to an appropriate healthcare facility, as determined by the healthcare provider, for at least 2 months following Elevidys infusion; **and**
18. Provider Attestation has been submitted indicating the Member has reviewed the FDA-Medication Guide with the Provider and has been counseled on the risk of acute liver failure or death.

NOTE: The Plan considers additional indications not listed above as experimental, unproven, or investigational and therefore not medically necessary given the lack of scientific evidence to support that the treatment is effective in improving health outcomes.

Limitations

- If approval criteria are met, the Plan may authorize ELEVIDYS for one treatment only per lifetime.
- The length of authorization is 90 days from date of approval or up to the member's 6th birthday, whichever comes first.
- The Plan will not cover ELEVIDYS for any diagnoses other than Duchene muscular dystrophy (DMD) with confirmed mutation in the DMD gene.
- All other uses including for additional diagnoses and those based on **age** and **ambulatory** status are considered experimental, unproven, or investigational and therefore not medically necessary.

- Elevidys not intended for use in pregnant women.
- Elevidys administration should be postponed in patients with acute liver disease until resolved or controlled.
- Elevidys will not be covered if the Member demonstrates clinical decompensation from time of authorization to time of infusion and no longer meets clinical coverage criteria.

Codes

The following code(s) require prior authorization:

Table 1: HCPCS Codes

HCPCS Codes	Description
J1413	INJECTION, DELANDISTROGENE MOXEPARVOVEC-ROKL, PER THERAPEUTIC DOSE

References:

1. Clinicaltrials.gov. An open-label, systemic gene delivery study using commercial process material to evaluate the safety of and expression from SRP-9001 in subjects with duchenne muscular dystrophy (ENDEAVOR) (NCT04626674). Available at**:** Study Details | A Gene Transfer Therapy Study to Evaluate the Safety of and Expression From Delandistrogene Moxeparvovec (SRP-9001) in Participants With Duchenne Muscular Dystrophy (DMD) | ClinicalTrials.gov. Accessed on November 30, 2023.
2. Mendell JR, Sahenk Z, Lehman K, et al. Assessment of systemic delivery of rAAVrh74.MHCK7.micro-dystrophin in children with duchenne muscular dystrophy: a nonrandomized controlled trial. JAMA Neurol. 2020 Sep 1; 77 (9): 1122 - 31.
3. Clinicaltrials.gov. A phase 3 multinational, randomized, double-blind, placebo-controlled systemic gene delivery study to evaluate the safety and efficacy of SRP-9001 in subjects with duchenne Muscular dystrophy (EMBARK) (NCT05096221). Available at: Study Details | A Gene Transfer Therapy Study to Evaluate the Safety and Efficacy of Delandistrogene Moxeparvovec (SRP-9001) in Participants With Duchenne Muscular Dystrophy (DMD) | ClinicalTrials.gov. Accessed on November 30, 2023.
4. Elevidys [package insert]. Cambridge, MA: Sarepta Therapeutics, Inc.; November 2025.
5. Markati T, Oskoui M, Farrar MA, Duong T, Goemans N, Servais L. Emerging therapies for Duchenne muscular dystrophy. Lancet Neurol. 2022;21(9):814-829. doi:10.1016/S1474-4422(22)00125-9.
6. Darras B. Duchenne and Becker muscular dystrophy: Glucocorticoid and disease-modifying treatment. UpToDate.com/login [via subscription only]. Published August 7, 2024. Accessed on March 24, 2025.
7. Mendell JR, Shieh PB, McDonald CM, et al. Expression of SRP-9001 dystrophin and stabilization of motor function up to 2 years post-treatment with delandistrogene moxeparvovec gene therapy in individuals with Duchenne muscular dystrophy. Front Cell Dev Biol. 2023;11:1167762. Published 2023 Jul 11. doi:10.3389/fcell.2023.1167762.
8. Mendell JR, Proud C, Zaidman CM, et al. Practical Considerations for Delandistrogene Moxeparvovec Gene Therapy in Patients With Duchenne Muscular Dystrophy. Pediatr Neurol. 2024;153:11-18. doi:10.1016/j.pediatrneurol.2024.01.003
9. Kumar SRP, Duan D, Herzog RW. Immune Responses to Muscle-Directed Adeno-Associated Viral Gene Transfer in Clinical Studies. Hum Gene Ther. 2023;34(9-10):365-371. doi:10.1089/hum.2023.056.
10. Zaidman CM, Proud CM, McDonald CM, et al. Delandistrogene Moxeparvovec Gene Therapy in Ambulatory Patients (Aged ≥4 to <8 Years) with Duchenne Muscular Dystrophy: 1-Year Interim Results from Study SRP-9001-103 (ENDEAVOR). Ann Neurol. 2023;94(5):955-968. doi:10.1002/ana.26755.
11. Zhou T. Statistical review – Elevidys. FDA website. 2024. Accessed December 1, 2025 at https://www.fda.gov/media/179489/download?attachment
12. Oskoui M, Caller TA, et. al. Delandistrogene Moxeparvovec Gene Therapy in Individuals With Duchenne Muscular Dystrophy: Evidence in Focus: Report of the AAN Guidelines Subcommittee. Neurology. 2025 Jun 10;104(11):e213604. doi: 10.1212/WNL.0000000000213604. Epub 2025 May 14. Erratum in: Neurology. 2025 Aug 26;105(4):e214014. doi: 10.1212/WNL.0000000000214014. PMID: 40367405.

Approval And Revision History

December 20, 2023: Reviewed by the Medical Policy Approval Committee (MPAC), effective January 1, 2024

Subsequent endorsement date(s) and changes made:

- January 17, 2024: Reviewed by MPAC. Removed MATogether as applicable LOB, coverage moved to Unified Policies MNG effective March 4, 2024
- August 30, 2024: Reviewed by MPAC. Minor editorial refinements to Overview and FDA approved indications. Clarifying edits made to both Coverage Criteria and Limitation sections. Intent unchanged effective September 1, 2024
- December 18, 2024: Reviewed by MPAC. Limitation added stating authorization up to 90 days or Member's 6th birthday. Criteria unchanged. Effective February 1, 2025.
- April 16, 2025: Reviewed by MPAC. Updated references, effective May 16, 2025.
- December 17, 2025: Reviewed by MPAC. Guideline updated to reflect updated FDA packaging instructions. Changes

include additional language added to Overview Section to include black box safety warning and Point32Health requirements that providers comply with the FDA updates. Revisions to Criteria section to include documentation of member's ambulatory status, as well as confirmation of no hepatic impairment. In addition, criteria points # 10, 11, 13, 15, 17, and 18 added. Effective date February 1, 2026.

- May 20, 2026: Reviewed by MPAC, renewed without changes, references updated, effective July 1, 2026.

Background, Product and Disclaimer Information

Medical Necessity Guidelines are developed to determine coverage for benefits and are published to provide a better understanding of the basis upon which coverage decisions are made. We make coverage decisions using these guidelines, along with the Member's benefit document, and in coordination with the Member's physician(s) on a case-by-case basis considering the individual Member's health care needs.

Medical Necessity Guidelines are developed for selected therapeutic or diagnostic services found to be safe and proven effective in a limited, defined population of patients or clinical circumstances. They include concise clinical coverage criteria based on current literature review, consultation with practicing physicians in our service area who are medical experts in the field, FDA and other government agency policies, and standards adopted by national accreditation organizations. We revise and update Medical Necessity Guidelines annually, or more frequently if new evidence becomes available that suggests needed revisions.

For self-insured plans, coverage may vary depending on the terms of the benefit document. If a discrepancy exists between a Medical Necessity Guideline and a self-insured Member's benefit document, the provisions of the benefit document will govern. For Tufts Health Together (Medicaid), coverage may be available beyond these guidelines for pediatric members under age 21 under the Early and Periodic Screening, Diagnostic and Treatment (EPSDT) benefits of the plan in accordance with 130 CMR 450.140 and 130 CMR 447.000, and with prior authorization.

Treating providers are solely responsible for the medical advice and treatment of Members. The use of this guideline is not a guarantee of payment, or a final prediction of how specific claim(s) will be adjudicated. Claims payment is subject to eligibility and benefits on the date of service, coordination of benefits, referral/authorization, utilization management guidelines when applicable, and adherence to plan policies, plan procedures, and claims editing logic.