Reference number(s)
6032-A

Specialty Guideline Management Elevidys

Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

Brand Name	Generic Name
Elevidys	delandistrogene moxeparvovec-rokl

Indications

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indications[1]

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.

The DMD indication in non-ambulatory patients is approved under accelerated approval based on expression of Elevidys microdystrophin. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial(s).

All other indications are considered experimental/investigational and not medically necessary.

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Documentation

Submission of the following information is necessary to initiate the prior authorization review:

- Genetic test results confirming the DMD diagnosis.
- Medical records (e.g., chart notes and/or laboratory reports) documenting following:
    - Ambulation status
    - Prior use of corticosteroids or a documented contraindication or intolerance
    - Baseline liver function, platelet count, and troponin-I levels

Prescriber Specialties

This medication must be prescribed by or in consultation with a physician who specializes in the treatment of Duchenne muscular dystrophy (DMD) (e.g., pediatric neurologist, neuromuscular specialist).

Exclusions

- Coverage will not be provided for members with a deletion in exon 8 and/or exon 9 in the DMD gene.
- Elevidys will not be used in combination with exon-skipping therapies (e.g., casimersen, eteplirsen, golodirsen, viltolarsen).

Coverage Criteria

Duchenne Muscular Dystrophy[1-6]

Authorization of 3 months for one dose total may be granted for treatment of Duchenne muscular dystrophy when all of the following criteria are met:

- Member is male.
- Member is 4 - 20 years of age.
- Member has a definitive diagnosis of DMD confirmed via genetic testing.
- Member meets either of the following criteria:
    - Member is ambulatory (e.g., able to walk with or without assistance, not wheelchair dependent).
    - Member is non-ambulatory and has a Performance Upper Limb (PUL) entry item score of at least 3 and a total PUL score of 20 – 40.
- Member has anti-recombinant adeno-associated virus serotype rh74 (anti-AAVrh74) total binding antibody titers of < 1:400.

- Member does not currently have an active infection.
- Member has been on a stable dose of corticosteroids (e.g., prednisone) for at least 12 weeks prior to and following receipt of Elevidys infusion unless contraindicated or not tolerated.
- Member does not have signs of cardiomyopathy (e.g., ejection fraction < 40%).
- Liver function, platelet count, and troponin-I levels have been assessed at baseline and will be monitored as clinically appropriate.
- Member has not received treatment with Elevidys previously.

References

1. Elevidys [package insert]. Cambridge, MA: Sarepta Therapeutics, Inc.; June 2024.
2. Muntoni F, Murcuri E, Schmidt UK, et al. EMBARK, a Phase 3 Trial Evaluating Safety and Efficacy of Delandistrogene Moxeparvovec (SRP9001) in Duchenne Muscular Dystrophy (DMD): Study Design and Baseline Characteristics (P5-8.012). Neurology Apr 2023, 100 (17 Supplement 2) 3691.
3. ClinicalTrials.gov. NCT05096221 (Embark, Study 301). A gene transfer therapy to evaluate the safety and efficacy of Delandistrogene Moxeparvovec (SRP-9001) in Participants with Duchenne Muscular Dystrophy. Accessed September 9, 2024.
4. Muntoni F, Murcuri E, McDonald C. ENVISION, a Phase 3 Randomized Trial Evaluating Safety and Efficacy of Delandistrogene Moxeparvovec (SRP- 9001) in Duchenne Muscular Dystrophy (DMD): Study Design. Presented at the World Muscle Society, Charleston, USA; 3-7 October, 2023. P.47.
5. 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;77(9):1122-1131.
6. 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 Nov;94(5):955-968.