Draft Revised Policy: Do Not Implement

Delandistrogene moxeparvovec-rokl (Elevidys®)

IMPORTANT REMINDER

We develop Medical Policies to provide guidance to Members and Providers. This Medical Policy relates only to the services or supplies described in it. The existence of a Medical Policy is not an authorization, certification, explanation of benefits or a contract for the service (or supply) that is referenced in the Medical Policy. For a determination of the benefits that a Member is entitled to receive under his or her health plan, the Member's health plan must be reviewed. If there is a conflict between the medical policy and a health plan or government program (e.g., TennCare), the express terms of the health plan or government program will govern.

The proposal is to add text/statements in red and to delete text/statements with strikethrough:

POLICY

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 Indication

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.

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 ambulation status. ~~the following:~~
  1. ~~Ambulation status~~
  2. ~~Prior use of corticosteroids or a documented contraindication or intolerance~~
  3. ~~Baseline liver function, platelet count, and troponin I levels~~

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).~~

~~**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).~~

COVERAGE CRITERIA ~~FOR INITIAL APPROVAL~~

Duchenne Muscular Dystrophy

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 at least 4-20 years of age.~~
- Member has a ~~definitive~~ diagnosis of DMD with a confirmed mutation in the DMD gene ~~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 4 through 5 years of age (at least 4 years 0 days and less than 6 years old)
- ~~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 does not have significant liver dysfunction or disease, defined as at least one of the following:
  - Preexisting liver impairment; or
  - Chronic hepatic condition; or
  - Acute liver disease (e.g., acute hepatic viral 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.~~

APPLICABLE TENNESSEE STATE MANDATE REQUIREMENTS

BlueCross BlueShield of Tennessee's Medical Policy complies with Tennessee Code Annotated Section 56-7-2352 regarding coverage of off-label indications of Food and Drug Administration (FDA) approved drugs when the off-label use is recognized in one of the statutorily recognized standard reference compendia or in the published peer-reviewed medical literature.

ADDITIONAL INFORMATION

For appropriate chemotherapy regimens, dosage information, contraindications, precautions, warnings, and monitoring information, please refer to one of the standard reference compendia (e.g., the NCCN Clinical Practice Guidelines in Oncology (NCCN Guidelines®) published by the National Comprehensive Cancer Network®, Drugdex Evaluations of Micromedex Solutions at Truven Health, or The American Hospital Formulary Service Drug Information).

REFERENCES

1. Elevidys [package insert]. Cambridge, MA: Sarepta Therapeutics, Inc.; August 2024.
2. ClinicalTrials.gov. NCT03769116. A randomized, double-blind placebo-controlled study of delandistrogene moxeparvovec (SRP-9001) for Duchenne Muscular Dystrophy. Accessed May 9, 2025.
3. ~~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.~~
4. ~~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.~~
5. ~~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.~~
6. ~~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.~~
7. 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

EFFECTIVE DATE

ID_CHS