

Elevidys® (delandistrogene moxeparvovec-rokl)

When requesting Elevidys (delandistrogene moxeparvovec-rokl), the individual requiring treatment must be diagnosed with the following FDA-approved indication and meet the specific coverage guidelines and applicable safety criteria for the covered indication.

FDA-approved indication

Elevidys is an adeno-associated virus vector-based gene therapy 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 confirmatory trials.

Coverage Guidelines

The prescribing information for Elevidys states that a clinical benefit has not been established. Due to the lack of clinical efficacy data, approval is not recommended for Elevidys.

References

1. Elevidys® intravenous infusion [prescribing information]. Cambridge, MA: Sarepta Therapeutics, Inc.; June 2023.
2. US Food and Drug Administration. Cellular, Tissue, and Gene Therapies Advisory Committee Meeting. May 12, 2023. Available at: <https://www.fda.gov/advisory-committees/advisory-committee-calendar/cellular-tissue-and-gene-therapies-advisory-committee-may-12-2023-meeting-announcement-05122023> Accessed on May 10, 2023.
3. Sarepta Therapeutics, Inc. Sponsor Briefing Document. Cellular, Tissue, and Gene Therapies Advisory Committee Meeting. May 12, 2023. Available at: <https://www.fda.gov/advisory-committees/advisory-committee-calendar/cellular-tissue-and-gene-therapies-advisory-committee-may-12-2023-meeting-announcement-05122023> Accessed on May 10, 2023.
4. 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.* 11;1167762. DOI: 10.3389/fcell.2023.1167762.
5. 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.
6. Birnkrant DJ, Bushby K, Bann CM, et al. Diagnosis and management of Duchenne muscular dystrophy, part 2: respiratory, cardiac, bone health, and orthopaedic management. *Lancet Neurol.* 2018;17(4):347-361.
7. Birnkrant DJ, Bushby K, Bann CM, et al. Diagnosis and management of Duchenne muscular dystrophy, part 3: primary care, emergency medicine, psychological care, and transitions of care across the lifespan. *Lancet Neurol.* 2018;17(5):445-455.