Elevidys (delandistrogene moxeparvovec-rokl)

Disclaimer

Clinical guidelines are developed and adopted to establish evidence-based clinical criteria for utilization management decisions. Clinical guidelines are applicable according to policy and plan type. The Plan may delegate utilization management decisions of certain services to third parties who may develop and adopt their own clinical criteria.

Coverage of services is subject to the terms, conditions, and limitations of a member's policy, as well as applicable state and federal law. Clinical guidelines are also subject to in-force criteria such as the Centers for Medicare & Medicaid Services (CMS) national coverage determination (NCD) or local coverage determination (LCD) for Medicare Advantage plans. Please refer to the member's policy documents (e.g., Certificate/Evidence of Coverage, Schedule of Benefits, Plan Formulary) or contact the Plan to confirm coverage.

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Summary

Duchenne muscular dystrophy (DMD) is an X-linked genetic disorder characterized by progressive muscle weakness and wasting due to lack of dystrophin protein. It primarily affects males, with onset around 2-5 years of age. As the disease progresses, individuals experience loss of ambulation, respiratory and cardiac complications, and premature death. There is no cure for DMD. Current management focuses on glucocorticoids to slow progression, supportive care, and emerging gene targeted therapies such as exon skipping therapies (e.g., Exondys 51 [eteplirsen], Vyondys 53 [golodirsen], Viltepso [viltolarsen], and Amondys 45 [casimersen]).

Elevidys (delandistrogene moxeparvovec-rokl) is an adeno-associated virus (AAV)-based gene therapy designed to deliver a microdystrophin transgene to muscle cells. It was originally approved by theFDA under the accelerated approval pathway for:

- Ambulatory pediatric patients aged 4-5 years with confirmed DMD gene mutations.
- Non-ambulatory patients aged 4 years and older with confirmed DMD gene mutations (approval contingent on confirmatory trials).

In June of 2024 it received traditional approval for ambulatory patients 4 years of age and older withDMD.

While Elevidys (delandistrogene moxeparvovec-rokl) shows promise in increasing microdystrophin expression, its clinical efficacy in improving functional outcomes remains under investigation.

In July, 2025, the United States Food and Drug Administration (FDA) requested that the manufacturers of Elevidys (delandistrogene moxeparvovec-rokl) voluntarily suspend the distribution of this product and place clinical trials on hold following three deaths potentially related to this product. The three deaths may have been the result of acute liver failure, occurring after the administration of Elevidys (delandistrogene moxeparvovec-rokl) or an investigational gene therapy that uses the same AAVrh74 serotype that is used in Elevidys (delandistrogene moxeparvovec-rokl). At the same time, the FDA also removed this manufacturers Platform Technology designation for AAVrh74 due to this safety concern. This hold was subsequently removed by the FDA at the end of July, 2025, for ambulatory individuals; however, a hold remains for those who are non-ambulatory.

In November 2025, the FDA added a boxed warning to Elevidys (delandistrogene moxeparvovec-rokl) for the risk of acute serious liver injury, including life-threatening and fatal acute liver failure. The boxed warning also states that those with preexisting liver impairment may be at higher risk; pre-infusion liver function assessment should be conducted in anyone undergoing Elevidys (delandistrogene moxeparvovec-rokl). Systemic corticosteroids should be administered before and after Elevidys (delandistrogene moxeparvovec-rokl) administration reduce this risk, with at least three (3) months of close liver function monitoring (of which the individual should maintain close proximity to appropriate healthcare facilities for the first two [2] months), and specialists should be consulted upon any suspected acute serious liver injury or failure.

Definitions

"Acute liver failure" is a rapid progressing condition in which the liver loses function causing symptoms such as nausea, jaundice, or confusion. Acute liver failure can be fatal, especially if not addressed promptly by trained healthcare professionals in an appropriate setting.

"Accelerated approval" is a pathway by the FDA to allow earlier approval of drugs that treat serious conditions and fill an unmet medical need, based on surrogate or intermediate endpoints.

"Adeno-associated virus (AAV)" is a type of virus that can be used to deliver genetic material into cells during gene therapy.

"Ambulation" refers to the ability to walk without assistance.

"Anti-AAV antibodies" are proteins produced by the immune system in response to the AAV vector used in some gene therapies. These can potentially limit the effectiveness of future AAV-based gene therapies.

"Biomarkers" are measurable indicators of the severity or presence of a disease.

"Blinded studies" are clinical trials in which participants do not know whether they are receiving the experimental treatment or a placebo.

"Duchenne muscular dystrophy (DMD)" is a genetic disorder characterized by progressive muscle degeneration and weakness. It is caused by an absence of dystrophin, a protein that helps keep muscle cells intact.

"Dystrophin" is a protein necessary for muscle strength and function. Its absence or dysfunction leads to the muscle degeneration seen in DMD.

"Gene therapy" is a technique that aims to treat or prevent disease by replacing, adding, or editing genes within an individual's cells.

"Glucocorticoids" is a class of steroid hormones that can reduce inflammation and alter the immune response. They are used in DMD to slow the progression of the disease.

"Micro-dystrophin" is a smaller but functional form of the dystrophin protein, which gene therapies like Elevidys aim to introduce to patients' muscles.

"North Star Ambulatory Assessment (NSAA)" is a validated measure to assess ambulatory function in DMD patients.

"Open-label" is a type of clinical trial where both the researchers and the participants know which treatment is being administered.

"Placebo-controlled trial" is a study where the effect of a drug is compared with a placebo (a substance with no therapeutic effect).

"Primary & Secondary endpoints" are specific outcomes that a clinical trial is designed to measure, determining the effectiveness and side effects of a treatment.

"Randomized controlled trials" are clinical studies where participants are assigned to groups randomly, with one group receiving the treatment and the other a control (like a placebo).

"X-linked" is a mode of inheritance where the gene causing the condition is located on the X chromosome.

Policy Statement on Elevidys (delandistrogene moxeparvovec-rokl) Efficacy Information

There is insufficient evidence to indicate that Elevidys (delandistrogene moxeparvovec-rokl) provides clinically meaningful benefits that outweigh the potential risks for those with Duchenne muscular dystrophy (DMD). The Plan considers Elevidys (delandistrogene moxeparvovec-rokl) to be not medically necessary for DMD at this time, as it is deemed to be experimental, investigational, or unproven.

- The clinical trials for Elevidys (delandistrogene moxeparvovec-rokl) (e.g., SRP-9001-101, SRP-9001-102, SRP-9001-103) have not demonstrated consistent, statistically significant improvements in functional outcomes, such as the North Star Ambulatory Assessment (NSAA), which is a key measure of efficacy in Duchenne muscular dystrophy (DMD).
    - Trial #1: SRP-9001-101 (Study 101, NCT03375164) an open-label, phase 1/2a, non-randomized controlled study with small sample size (n=4), showing numerical improvements in NSAA scores but lacking robust statistical significance or comparator analysis A follow-up of this study found sustained improvement in NSAA scores at year four (4).
    - Trial #2: SRP-9001-102 (Study 102, NCT03769116), a phase-2, double-blind, two-part cross-over study, failed to demonstrate statistically significant improvement in NSAA scores at 48 weeks compared to placebo. Subgroup analyses suggested potential benefit in patients aged 4-5 years.
    - Trial #3: SRP-9001-103 (Study 103, NCT04626674) is an ongoing open-label study showing increased micro-dystrophin expression but limited functional data.
    - Trial #4: SRP-9001-301(NCT05096221) is a completed phase 3, multinational, randomized, double-blind, placebo-controlled study of 126 individuals (ages 4 to 8 years at randomization) with ambulatory DMD. The primary endpoint of change from baseline NSAA did not meet statistical significance at week 52. No secondary endpoints met statistical significance including microdystrophin expression, time to rise, 10-meter walk/run, stride velocity, 100-meter walk/run, time to ascend 4 steps, PROMIS mobility and upper extremity, and number of NSAA skills gained/improved. A qualitative study of caregivers using in-trial semi-structured interviews found that caregivers indicated that "no change," "minimal," or 1-point change in improvement in Caregiver Global Impression measures of Change (CaGI-C) or Severity (CaGI-S) would be meaningful. In an exploratory analysis of this study, CaGI-C had a treatment difference of 1.7 (95% CI:

0.90, 2.5) and CaGI-S difference of 1.1 (95% CI: 0.30, 1.9) favoring Elevidys (delandistrogene moxeparvovec-rokl).
    - On-going studies include:
        - A phase 3 multinational long-term follow-up study of those who have previously received Elevidys (delandistrogene moxeparvovec-rokl) (NCT05967351) including 400 participants (enrolled by invitation).
        - Phase 1 open-label study evaluation of the safety and expression of Elevidys (delandistrogene moxeparvovec-rokl) in those with DMD (NCT04626674) including 55 participants (active, not recruiting).
        - A two-part open-label study assess Elevidys (delandistrogene moxeparvovec-rokl) in those with DMD and under the age of 4 (NCT06128564) - an estimated enrollment of 21 participants (currently recruiting).
        - A phase 3 multinational, randomized, double-blind, placebo-controlled study of Elevidys (delandistrogene moxeparvovec-rokl) evaluating safety and efficacy in those with non-ambulatory DMD (NCT05881408) - and estimated 148 participants (active, not recruiting).
        - An open-label study of the safety, efficacy, tolerability and SRP-9001 expression in those receiving Elevidys (delandistrogene moxeparvovec-rokl) followed by imlifidase infusion in those with DMD and pre-existing antibodies to rAAVrh74 (NCT06241950) with an estimated enrollment of 6 participants (enrollment by invitation).
        - A long-term multicenter prospective observation study assessing comparative effectiveness and safety of Elevidys (delandistrogene moxeparvovec-rokl) compared to usual care (NCT06270719) - an estimated enrollment of 500 participants (enrollment by invitation).
- A systematic review and meta-analysis of five (5) trials including 190 participants, at one-year a significant difference was found on the NSAA (2.63 points, 95% CI: 1.74, 3.52) and supine to stand test (-0.29 second, 95% CI: -0.52, -0.06 seconds) compared to placebo (-0.64 seconds, 95% CI: -0.99, -0.30 seconds). No other outcomes were found to be statistically significant (including climb 4 stairs, 100-meter timed walk test, 10-meter run test).
- One cohort study of 11 participants showed an improvement of motor function and a sustained 4-point improvement in NSAA scores at one-year. It was noted that high doses of corticosteroids may have confounded the results, and there was no available comparator group.
- While Elevidys (delandistrogene moxeparvovec-rokl) has shown an increase in micro-dystrophin expression (a surrogate biomarker), there is no established correlation between this biomarker and clinically meaningful functional improvements in patients with DMD.
- Subgroup analyses suggest potential benefits in younger patients (ages 4-5 years), but these findings are exploratory and not prespecified, limiting their reliability.
- Safety Considerations
    - Serious adverse events, including acute liver injury, immune-mediated myositis, and myocarditis, have been observed in clinical trials. In November, 2025, the FDA added a

boxed warning to Elevidys (delandistrogene moxeparvovec-rokl) regarding the risk of acute serious liver injury, including life-threatening and fatal acute liver failure. Those with preexisting liver impairment may be at higher risk; pre-infusion liver function assessment should be conducted in anyone undergoing Elevidys (delandistrogene moxeparvovec-rokl). Systemic corticosteroids should be administered before and after Elevidys (delandistrogene moxeparvovec-rokl) administration reduce this risk, with at least three (3) months of close liver function monitoring (of which the individual should maintain close proximity to appropriate healthcare facilities for the first two [2] months), and specialists should be consulted upon any suspected acute serious liver injury or failure.
    - The long-term safety profile of Elevidys (delandistrogene moxeparvovec-rokl) remains uncertain, particularly regarding the implications of high anti-AAVrh74 antibody titers, which may limit future gene therapy options.
    - A voluntary hold was recommended by the FDA (and subsequently removed for those with ambulatory DMD) in July of 2025 for the administration of Elevidys (delandistrogene moxeparvovec-rokl) following three (3) deaths which may have been the result of acute liver failure, occurring after the administration of Elevidys (delandistrogene moxeparvovec-rokl) or an investigational gene therapy that uses the same AAVrh74 serotype that is used in Elevidys (delandistrogene moxeparvovec-rokl). Since this hold was placed, the FDA concluded that at least one (1) death was ruled to be unrelated to Elevidys (delandistrogene moxeparvovec-rokl). The voluntary hold remains for those with non-ambulatory DMD as the FDA continues to investigate these findings.
- Elevidys (delandistrogene moxeparvovec-rokl) was initially approved under the accelerated approval pathway based on micro-dystrophin expression as a surrogate endpoint. For ambulatory pediatric patients aged 4-5 years, the FDA has granted traditional approval based on evidence of clinical benefit. However, for non-ambulatory patients aged 4 years and older, the approval remains under the accelerated pathway, contingent on confirmatory trials demonstrating clinical efficacy.
- Guidelines/Position statements:
    - In a report by the American Academy of Neurology, no recommendation was made regarding the use of Elevidys (delandistrogene moxeparvovec-rokl) for the management of DMD. This report includes a review of 6 studies, of which 4 had available peer-reviewed data (n=134). They noted that class I studies (studied with data comparing treatment and placebo groups) failed to meet statistical significance for NSAA change from baseline.
    - No guidelines on DMD have been updated since Elevidys (delandistrogene moxeparvovec-rokl) was originally approved in 2023.

Medical Necessity Criteria for Elevidys (delandistrogene moxeparvovec-rokl)

Elevidys (delandistrogene moxeparvovec-rokl) is considered not medically necessary for any indication, including for the treatment of Duchenne muscular dystrophy (DMD).

Experimental or Investigational / Not Medically Necessary

Elevidys (delandistrogene moxeparvovec-rokl) is considered not medically necessary for any indication, including for the treatment of Duchenne muscular dystrophy (DMD) at this time. The available data are insufficient to demonstrate that Elevidys (delandistrogene moxeparvovec-rokl) provides clinically meaningful benefits that outweigh its risks.

The Plan will continue to monitor ongoing and future clinical trials, particularly confirmatory studies required by the FDA, to reassess its position as new evidence emerges. Individual coverage requests for Elevidys (delandistrogene moxeparvovec-rokl) may be considered on a case-by-case basis, particularly for members who meet the FDA-approved criteria. Until then, we encourage our members to explore alternative treatment options with their healthcare providers.

Applicable Billing Codes

Table 1	
CPT/HCPCS Codes for Duchenne muscular dystrophy (DMD) considered experimental, investigational, or unproven:	
Code	*Description*
96365	Intravenous infusion, for therapy, prophylaxis, or diagnosis (specify substance or drug); initial, up to 1 hour
96366	Intravenous infusion, for therapy, prophylaxis, or diagnosis (specify substance or drug); each additional hour (List separately in addition to code for primary procedure)
96367	Intravenous infusion, for therapy, prophylaxis, or diagnosis (specify substance or drug); additional sequential infusion of a new drug/substance, up to 1 hour (List separately in addition to code for primary procedure)
96368	Intravenous infusion, for therapy, prophylaxis, or diagnosis (specify substance or drug); concurrent infusion (List separately in addition to code for primary procedure)
J1413	Injection, delandistrogene moxeparvovec-rokl, per therapeutic dose

Table 2	
ICD-10 diagnosis codes for Duchenne muscular dystrophy (DMD) considered NOT medically necessary with Table X (CPT/HCPCS) codes:	
Code	*Description*
G71.01	Duchenne or Becker muscular dystrophy

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Clinical Guideline Revision / History Information

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