Policy Number	**RX501.163**
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Delandistrogene moxeparvovec-rokl

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Related Policies (if applicable)
None

Disclaimer

Medical policies are a set of written guidelines that support current standards of practice. They are based on current generally accepted standards of and developed by nonprofit professional association(s) for the relevant clinical specialty, third-party entities that develop treatment criteria, or other federal or state governmental agencies. A requested therapy must be proven effective for the relevant diagnosis or procedure. For drug therapy, the proposed dose, frequency and duration of therapy must be consistent with recommendations in at least one authoritative source. This medical policy is supported by FDA-approved labeling and/or nationally recognized authoritative references to major drug compendia, peer reviewed scientific literature and generally accepted standards of medical care. These references include, but are not limited to: MCG care guidelines, DrugDex (IIa level of evidence or higher), NCCN Guidelines (IIb level of evidence or higher), NCCN Compendia (IIb level of evidence or higher), professional society guidelines, and CMS coverage policy.

Carefully check state regulations and/or the member contract.

Each benefit plan, summary plan description or contract defines which services are covered, which services are excluded, and which services are subject to dollar caps or other limitations, conditions or exclusions. Members and their providers have the responsibility for consulting the member's benefit plan, summary plan description or contract to determine if there are any exclusions or other benefit limitations applicable to this service or supply. **If there is a discrepancy between a Medical Policy and a member's benefit plan, summary plan description or contract, the benefit plan, summary plan description or contract will govern.**

Legislative Mandates

EXCEPTION: For Illinois only: Illinois Public Act 103-0458 [Insurance Code 215 ILCS 5/356z.61] (HB3809 Impaired Children) states all group or individual fully insured PPO, HMO, POS plans amended, delivered, issued, or renewed on or after January 1, 2025 shall provide coverage for therapy, diagnostic testing, and equipment necessary to increase quality of life for children who have been clinically or genetically diagnosed with any disease, syndrome, or disorder that includes low tone neuromuscular impairment, neurological impairment, or cognitive impairment.

EXCEPTION: For HCSC members residing in the state of Ohio, § 3923.60 requires any group or individual policy (Small, Mid-Market, Large Groups, Municipalities/Counties/Schools, State Employees, Fully-Insured, PPO, HMO, POS, EPO) that covers prescription drugs to provide for the coverage of any drug

approved by the U. S. Food and Drug Administration (FDA) when it is prescribed for a use recognized as safe and effective for the treatment of a given indication in one or more of the standard medical reference compendia adopted by the United States Department of Health and Human Services or in medical literature even if the FDA has not approved the drug for that indication. Medical literature support is only satisfied when safety and efficacy has been confirmed in two articles from major peer-reviewed professional medical journals that present data supporting the proposed off-label use or uses as generally safe and effective. Examples of accepted journals include, but are not limited to, Journal of American Medical Association (JAMA), New England Journal of Medicine (NEJM), and Lancet. Accepted study designs may include, but are not limited to, randomized, double blind, placebo controlled clinical trials. Evidence limited to case studies or case series is not sufficient to meet the standard of this criterion. Coverage is never required where the FDA has recognized a use to be contraindicated and coverage is not required for non-formulary drugs.

Coverage

Delandistrogene moxeparvovec-rokl (Elevidys) **may be considered medically necessary** when **ALL** the following criteria are met:

1. Individuals ≥ 4 years of age; AND
2. Individual has a diagnosis of Duchenne muscular dystrophy (DMD) confirmed by a mutation in the DMD gene; AND
3. Submitted genetic testing for DMD confirms the individual does not have a deletion in exon 8 and/or 9; AND
4. Individual is ambulatory.

Delandistrogene moxeparvovec-rokl (Elevidys) for the treatment of all other non-Food and Drug Administration approved indications **is considered experimental, investigational and/or unproven**.

Policy Guidelines

None.

Description

Duchenne Muscular Dystrophy

Duchenne muscular dystrophy (DMD) is an X-linked, recessive disorder that occurs in approximately 1 in every 3500 to 5000 males. (2) It primarily affects males. However, females are also affected, but are usually asymptomatic. Even when symptomatic, most females typically only present with a mild form of the disease. According to U.S. epidemiologic data, the first signs or symptoms of DMD are noted at a mean age of 2.5 years (range, 0.2 to 1 years). Although histologic and laboratory evidence of myopathy may be present at birth, the clinical onset of skeletal muscle weakness usually does not become evident until early childhood. The average age at diagnosis is approximately 5 years. (3) Symptoms include motor difficulties such as difficulty running, jumping, and walking up stairs, along with an unusual waddling gait. Some

improvement in symptoms may be seen from 3 to 6 years of age, though gradual deterioration resumes, and most individuals lose ambulation by age 12 and require noninvasive ventilation by the late teenage years. Individuals progress from needing noninvasive ventilation only during night sleeping, followed by noninvasive ventilation during day and night sleeping, and then noninvasive ventilation during day and night over the course of 5 to 10 years. Median life expectancy more recently has increased into the fourth decade, primarily through improved respiratory management and cardiac care.

DMD occurs as a result of variant(s) in the gene responsible for producing dystrophin, a cohesive protein that is essential for maintaining muscle support and strength. *DMD* is the longest known human gene, and several variants can cause DMD. Most deletion variants disrupt the translational reading frame in the dystrophin messenger RNA resulting in an unstable, nonfunctional dystrophin molecule. As a result, there is progressive muscle degeneration leading to loss of independent ambulation, as well as other complications, including respiratory and cardiac complications. (4) Genetic testing is required to determine the specific *DMD* gene variant(s) for a definitive diagnosis, even when the absence of dystrophin protein expression has been confirmed by muscle biopsy. There are over 4700 variants in the Leiden DMD mutation database, and the most common variants are concentrated between exons 45 and 53.

Regulatory Status

In June 2023, delandistrogene moxeparvovec-rokl (Elevidys; Sarepta Therapeutics) was approved by the U.S. Food and Drug Administration (FDA) for treatment of ambulatory pediatric patients aged 4 through 5 years with DMD with a confirmed mutation in the *DMD* gene. This indication was approved under accelerated approval based on expression of delandistrogene moxeparvovec-rokl micro-dystrophin in skeletal muscle observed in patients treated with delandistrogene moxeparvovec-rokl. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial(s).

In June 2024, the FDA expanded the approval of delandistrogene moxeparvovec-rokl (Elevidys; Sarepta Therapeutics) for ambulatory and non-ambulatory individuals 4 years of age and older with DMD with a confirmed mutation in the *DMD* gene. It received a traditional approval in ambulatory individuals 4 years of age and older with DMD with a confirmed mutation in the *DMD* gene, and accelerated approval in non-ambulatory individuals 4 years of age and older with DMD with a confirmed mutation in the *DMD* gene.

On November 14, 2025, Sarepta Therapeutics announced an update to the prescribing information for Elevidys. The Elevidys label now includes several key updates (5):

- A boxed warning for the risk of acute serious liver injury (ALI) and acute liver failure (ALF).
- The non-ambulatory indication has been removed from the Indication and Usage section of the Prescribing Information.
- Expanded guidance for prescribers, including a modified pre- and post-infusion oral corticosteroids regimen, and enhanced monitoring recommendations on a weekly basis for 3 months post-infusion.

- A new Warnings & Precaution regarding increased susceptibility to serious infections due to immunosuppression.

Rationale

Medical policies assess the clinical evidence to determine whether the use of a technology improves the net health outcome. Broadly defined, health outcomes are length of life, quality of life, and ability to function including benefits and harms. Every clinical condition has specific outcomes that are important to individuals and to managing the course of that condition. Validated outcome measures are necessary to ascertain whether a condition improves or worsens; and whether the magnitude of that change is clinically significant. The net health outcome is a balance of benefits and harms.

To assess whether the evidence is sufficient to draw conclusions about the net health outcome of a technology, 2 domains are examined: the relevance and the quality and credibility. To be relevant, studies must represent one or more intended clinical use of the technology in the intended population and compare an effective and appropriate alternative at a comparable intensity. For some conditions, the alternative will be supportive care or surveillance. The quality and credibility of the evidence depend on study design and conduct, minimizing bias and confounding that can generate incorrect findings. The randomized controlled trial (RCT) is preferred to assess efficacy; however, in some circumstances, nonrandomized studies may be adequate. Randomized controlled trials are rarely large enough or long enough to capture less common adverse events and long-term effects. Other types of studies can be used for these purposes and to assess generalizability to broader clinical populations and settings of clinical practice.

Gene Therapy for Treatment of Duchenne Muscular Dystrophy

Clinical Context and Therapy Purpose

The purpose of delandistrogene moxeparvovec-rokl in individuals with Duchenne muscular dystrophy (DMD) who have a confirmed variant of the *DMD* gene is to provide a treatment option that is an alternative to or an improvement on existing therapies.

The following PICO was used to select literature to inform this policy.

Populations

The relevant population of interest is individuals with a confirmed variant of the *DMD* gene.

Interventions

The therapy being considered is delandistrogene moxeparvovec-rokl. It is an adeno-associated virus vector-based gene therapy which encodes a novel, engineered protein micro-dystrophin protein. This novel micro-dystrophin protein is a shortened version (138 kDa, compared to 427 kDa size of dystrophin expressed in normal muscle cells) that contains selected domains of dystrophin expressed in normal muscle cells. The novel microdystrophin produced after

administration of single dose gene therapy has been demonstrated to localize to the sarcolemma.

Treatment with delandistrogene moxeparvovec-rokl is intended to slow or stabilize progression of DMD, to alter the disease trajectory to a milder, Becker muscular dystrophy-like phenotype. Becker muscular dystrophy is similar to DMD, except that in Becker, symptoms begin later and progress at a slower rate.

Comparators
There is no cure for DMD. The following practice is currently being used to treat individuals with a confirmed variant of the *DMD* gene: standard multidisciplinary care including pharmacotherapy. Pharmacotherapy primarily involves corticosteroids (prednisone or deflazacort) for all individuals regardless of the genetic variant. Treatment is initiated once individuals reach a plateau of motor skill development, generally at ages 4 to 6 years, but before the onset of motor decline. The goal of corticosteroid therapy is to preserve ambulation and minimize respiratory, cardiac, and orthopedic complications. In addition, muscle weakness and pain, cardiac, pulmonary, orthopedic, and endocrine symptoms should be managed. (2)

Four antisense oligonucleotides—eteplirsen, golodirsen, viltolarsen, and casimersen have been approved by the U.S. Food and Drug Administration (FDA) for the treatment of DMD via the Accelerated Approval pathway. Each targets a specific exon. For example, eteplirsen targets skipping of exon 51, golodirsen and viltolarsen target skipping of exon 53, and casimersen targets skipping of exon 45. In each case, approval was based on the surrogate endpoint of expression of internally truncated dystrophin protein. The clinical benefit of all 4 of these drugs remains to be verified.

Outcomes
The general outcomes of interest are a change in disease status, functional outcomes, quality of life, treatment-related mortality, and treatment-related morbidity. See Table 1 for the description and relevance of specific outcome measures considered in this policy.

As per the FDA guidance document for developing drugs for the treatment of dystrophinopathies, the FDA has no defined set of required or recommended clinical outcome measures to be used in clinical studies. The guidance states that manufacturers should propose and, if necessary, develop endpoints that can validly and reliably assess individuals with a wide spectrum of symptoms and disease stages. Further, it states, "The sponsor should include an assessment of multiple efficacy endpoints, when feasible, to characterize the breadth of effects on dystrophin-related pathologies, including skeletal, respiratory, and cardiac muscle function, even if the primary endpoint is only 1 of these measures." (6)

Table 1. Health Outcome Measures That May Be Relevant to Muscular Dystrophinopathies

Outcomes Measure	Description	Scale	Clinically Meaningful Difference/Comment

Griffiths scale of mental development	Comprehensive, child-friendly developmental measure for continuous use from birth to 6 years (72 months).	Consists of 2 sets of scales, 1 for each age group 0 to 2 years and 2 to 8 years.	Although used in DMD, this is a non-specific measure and its appropriateness to measure clinical efficacy for DMD has not been established.
Bayley scales of infant and toddler development (Third edition)	Designed to assess developmental functioning from 1 month to 42 months of age. Covers 5 domains: cognitive, language, motor, adaptive, and social-emotional development.	Composite scores are derived for cognitive, language, and motor development and scaled to a metric, with a mean of 100, standard deviation of 15, and range of 40 to 160.	Although used in DMD, this is a non-specific measure and its appropriateness to measure clinical efficacy for DMD has not been established.
North Star Ambulatory Assessment (NSAA) or an age-appropriate modified NSAA	Measures functional motor abilities. Appropriate for ambulatory children ages ≥3 years of age with DMD.	17-item scale that grades each activity from 0 (unable to achieve independently) to 2 (normal- no obvious modification of activity). Scores can range from 0 to 34. Higher scores indicate improvement. Also includes recording timed items such as the 10-meter timed walk/run test and time to rise from the floor (Gower's test). These times are not included in the global score	Healthy boys obtain scores of 34 by 4 years of age. Boys with DMD achieve peak score of 26 around age 6 years. (7)
6-minute walk test (6MWT) or shorter versions such as the 2-minute walk test	Measures strength and endurance, can be appropriate for individuals as young as 5 to 6 years of age. Performance may increase with	Assesses distance walked in 6 minutes.	Estimates of minimum clinically important difference for DMD individuals of a change of 30 meters have been reported. (8, 9)

	time in very young individuals whereas performance tends to worsen with time in older individuals. Floor effect of losing ambulation in older individuals with more advanced disease and analyses of change in 6MWT can be strongly influenced by the inclusion or exclusion of individuals who lose ambulation during the trial; such individuals contribute zero values.		Interpretation of 6MWT results is limited by the variability in testing procedures and individual motivation.
Myometric assessments	Appropriate to measure increase or preservation of muscle strength, and it can be used to provide reliable measurements in children ages 5 years and older.		Clinical meaningfulness of differences in muscle strength should be supported by the magnitude of the effect observed or by the demonstration of a drug effect on an appropriate functional measure.
Specific clinical respiratory outcomes	Nocturnal desaturation, aspiration pneumonia, and progression to mechanically assisted ventilation	Varied outcome measure (dichotomous or continuous)	Clinical meaningfulness of differences should be supported by the magnitude of the effect observed or by the demonstration of a drug effect on an appropriate functional measure
Biomarker (such as dystrophin or microdystrophin)	Deficiency of functional dystrophin is the proximate	Microdystrophin levels are measured in muscle fibers by	Microdystrophin expression can only be viewed as

	cause of the symptomatic and functional consequences of dystrophinopathies. Micro-dystrophin produced by cells transduced by delandistrogene moxeparvovec-rokl is a novel, engineered protein consisting of selected domains of the normal, full-length dystrophin protein.	immunohistochemical analysis to detect the presence or absence of microdystrophin regardless of the actual quantity of microdystrophin present while Western blot analysis quantifies the amount of microdystrophin in the muscle tissue sample and expressed as a percent of control (i.e., as a percent of levels of normal, wild-type dystrophin in muscle tissues of healthy individuals without DMD).	supportive of the proof of principle. It is currently uncertain how predictive of sustained functional improvement the detected microdystrophin level could be, and what levels may be required for a meaningful clinical improvement. Further, no epidemiologic or pathophysiologic evidence is available regarding the function of micro-dystrophin. The protein differs in important ways from both the endogenous shortened forms of dystrophin in individuals with Becker muscular dystrophy, and the internally truncated dystrophins expressed through exon-skipping drugs. Therefore, clinical effects of microdystrophin is still not fully known.

6MWT: 6-minute walk test; DMD: Duchenne muscular dystrophy; NSAA: North Star Ambulatory Assessment.

Study Selection Criteria

Methodologically credible studies were selected using the following principles:

- To assess efficacy outcomes, comparative controlled prospective trials were sought, with a preference for RCTs.

- In the absence of such trials, comparative observational studies were sought, with a preference for prospective studies.
- To assess long-term outcomes and adverse events, single-arm studies that capture longer periods of follow-up and/or larger populations were sought.
- Consistent with a 'best available evidence approach' within each category of study design, studies with larger sample sizes and longer durations were sought.
- Studies with duplicative or overlapping populations were excluded.

The clinical development program is summarized in Table 2 and consists of 5 interventional studies. Of these, the manufacturer submitted data from 3 clinical studies (Study 101, 102 and 103) for consideration to the FDA. The phase 3 randomized, double-blinded clinical trial (Study 303 or Embark) was the primary study to verify clinical benefit. (10)

Both the accelerated as well as subsequent traditional approval of delandistrogene moxeparvovec-rokl by the FDA was in contrast to the recommendations made by FDA internal review teams, which did not recommend approval based upon their overall evaluation of the data submitted. (11, 12) The initial decision for accelerated approval was based on a post-hoc subgroup exploratory analysis of NSAA total score in 16 individuals (of the total 41 in the Study 102) ages 4 through 5 years and also considered the use of biomarker data (delandistrogene moxeparvovec-rokl micro-dystrophin expression as measured by Western blot) from cohort 1 of Study 103 that included 20 ambulatory male individuals aged 4 through 7 years. (12) The subsequent decision for traditional approval was based on a statistically significant difference in 3 secondary efficacy endpoints, including time to rise from the floor, 10-meter walk/run (10-MWR) and time to ascend 4 steps even though the study failed to meet the primary endpoint of change in NSAA total score from baseline to week 52 post treatment. (11)

Table 2. Summary of the Clinical Development Program for Delandistrogene Moxeparvovec-rokl

Study	Study 101	Study 102 (Study 1 in label)	Study 103 (Endeavor, Study 2 in label)	Study 301 (Embark, Study 3 in label)	Study 303 (Envision)
NCT Number	NCT03375164	NCT03769116	NCT04626674	NCT05096221	NCT05881408
Phase	1	2	1	3	3
Study Population	Ambulatory boys with DMD, aged 4 to 7 years	Ambulatory boys with DMD, aged 4 to 7 years	Cohort 1: Ambulatory boys aged 4 to 7 years Cohort 2: Ambulatory boys aged 8 to 17 years Cohort 3:	Ambulatory boys with DMD, aged 4 to 7 years	Cohort 1: Non-ambulatory Cohort 2: Ambulatory and ≥8 to <18 years of age at the time of screening.

			Non-ambulatory boys, no age restriction Cohort 4: Ambulatory boys aged 3 to 4 years old		
Status	Completed and published (13, 14)	Ongoing and published (15)	Ongoing and published (16)	Ongoing and published (17, 18)	Ongoing
Study Dates	2018-2023	2018-2026	2020-2028	2021-2024	2023-2027
Design	Open-label, single-arm	Part 1: DBRCT; placebo-controlled (48 weeks) Part 2: Cross-over, blinding maintained (48 weeks) Part 3: Open-label follow-up (5 years)	Open-label, single-arm	Part 1: DBRCT; placebo-controlled Part 2: Cross-over, blinding maintained	Part 1: DBRCT; placebo-controlled Part 2: Cross-over, blinding maintained
Sample Size	4	41	48	126	148
Follow-Up	12 weeks	48 weeks	12 weeks	2 years	128 weeks

DMD: Duchenne muscular dystrophy; DBRCT: double-blind randomized controlled study; NCT: national clinical trial.

Pivotal Studies

Study characteristics, baseline patient characteristics, and results are summarized in Tables 3 to 6, respectively. Study 102 was the 48-week, randomized, double-blind, placebo-controlled trial that randomized 41 study participants 1:1 to receive either delandistrogene moxeparvovec-rokl (n=20) or placebo (n=21), as a single intravenous infusion via a peripheral limb. All participants were on a stable dose of corticosteroids for DMD for at least 12 weeks prior to infusion of the gene therapy, had baseline anti-AAVrh74 antibody titers <1:400 and received at least 1 mg/kg of a glucocorticoid (prednisone equivalent) daily in addition to their baseline stable oral corticosteroid dose beginning 1 day prior to infusion and for at least 60 days after the infusion. Randomization was stratified by age (i.e., aged 4 to 5 years vs. aged 6 to 7 years). In the delandistrogene moxeparvovec-rokl group, 8 participants received the FDA approved dose of 1.33×10^{14} vg/kg while 12 received lower doses. The part 1 of the study included the randomized, double-blind, placebo-controlled phase. Part 2 was the cross-over phase in which

participants who received delandistrogene moxeparvovec-rokl in part 1 were then administered placebo in part 2, and vice-versa. Unlike for cross-over studies with small-molecule drugs, no wash-out period is possible for gene therapies. Therefore, although the blind was maintained in part 2, by that point the subjects, caregivers, and evaluators were aware that all subjects had now received delandistrogene moxeparvovec-rokl, rendering part 2 effectively an open-label study.

Study 103 is an ongoing, open-label study that included 5 cohorts with total of 48 participants aged 3 through 20 years. Cohorts 3 and 5b included 8 non-ambulatory individuals. All participants received a single intravenous infusion of 1.33×10^{14} vg/kg delandistrogene moxeparvovec-rokl if they weighed less than 70 kg or 9.31×10^{15} vg/kg total fixed dose if they weighed 70 kg or greater. Majority of study participants (77%) were white with a mean age of 7.7 years (range: 3.2 to 20.2), mean weight of 30.1 kg (range: 12.5 to 80.1), mean North Star Ambulatory Assessment (NSAA) total score of 20.3 points (range: 11 to 30), and mean time to rise from floor of 4.7 seconds (range: 2.4 to 9.7). All study participants received corticosteroids for DMD before infusion and had baseline anti-AAVrh74 antibodies titers ≤1:400.

Study 301 is a multicenter, randomized, double-blind, placebo-controlled study in which 125 ambulatory male individuals aged 4 through 7 years received a single intravenous infusion of 1.33×10^{14} vg/kg delandistrogene moxeparvovec-rokl. The efficacy outcome measure of the study was to evaluate the effect of delandistrogene moxeparvovec-rokl on physical function as assessed by the NSAA total score. Key secondary outcome measures were to evaluate expression of micro-dystrophin in skeletal muscle, time to rise from floor, and time of 10-MWR.

Clinical Outcomes

Both 102 and 301 studies failed to show a statistically significant difference in the primary endpoint of change in the NSAA total score between the treated and the placebo group. In study 102, the least squares (LS) mean change in the NSAA total score from baseline to week 48 was 1.7 points for the delandistrogene moxeparvovec-rokl group and 0.9 points for the placebo group (p=.37). In study 301, the LS mean change in the NSAA total score from baseline to week 52 was 2.57 points for the delandistrogene moxeparvovec-rokl group and 1.92 points for the placebo group (p=.24). Thus, clinical benefit was not demonstrated in the primary efficacy endpoint of NSAA total score from baseline in either study. (1)

Data from studies 102 and 301 were not pooled for an efficacy analysis due to multiple reasons. (10) The study population in study 102 differed in age and genetic diagnosis of DMD eligibility criteria from study 301. In addition, study 102 used a study product that was not ready for commercial use and a dose that was different from study 301. A side by side comparison of main efficacy from the 2 randomized studies are summarized in Table 7. (10) Multiple inconsistencies in key secondary outcomes were observed between the 2 studies. For example, the key secondary endpoint of 10-MWR test showed an advantage for delandistrogene moxeparvovec-rokl in study 301 but an advantage for placebo in study 102.

Biomarker Data

Muscle biopsies were obtained at baseline prior to infusion of gene therapy and at week 12 in all study participants. The absolute amount of delandistrogene moxeparvovec-rokl micro-dystrophin in muscle biopsy tissue samples were measured by western blot assay, adjusted by muscle content, and expressed as a percent of control (i.e., as a percent of levels of normal, wild-type dystrophin in muscle tissues of healthy individuals). Micro-dystrophin expression in delandistrogene moxeparvovec-rokl-treated participants was the primary objective of study 103 and 102 and a key secondary objective for study 301. Results of participants receiving the FDA approved dose for delandistrogene moxeparvovec-rokl in study 103 and 102 are included in Table 6. In study 301, muscle biopsies were obtained in 31 individuals. For the delandistrogene moxeparvovec-rokl-treated participants (n=17), the mean micro-dystrophin expression at week 12 was 34.3% (±41.0%) compared to 0% in placebo-treated participants (n=14). (1)

Safety
The safety database consists of the 156 individuals with a confirmed mutation in the *DMD* gene who received a single intravenous infusion of delandistrogene moxeparvovec-rokl in 4 clinical studies. Of these, 144 individuals received the intended dose of delandistrogene moxeparvovec-rokl (1.33×10^{14} vg/kg). No deaths were reported. In clinical trials, immune-mediated myositis was observed approximately 1 month following delandistrogene moxeparvovec-rokl infusion in individuals with deletion mutations involving exon 8 and/or exon 9 in the DMD gene. Symptoms of severe muscle weakness, including dysphagia, dyspnea and hypophonia, were observed. In a life-threatening case of immune-mediated myositis, symptoms resolved during hospitalization following additional immunomodulatory treatment; muscle strength gradually improved but did not return to baseline level. These immune reactions may be due to a T-cell based response from lack of self-tolerance to a specific region encoded by the transgene corresponding to exons 1-17 of the DMD gene. Limited data are available for delandistrogene moxeparvovec-rokl treatment in individuals with mutations in the DMD gene in exons 1 to 17 and/or exons 59 to 71. Individuals with deletions in these regions may be at risk for a severe immune-mediated myositis reaction. Delandistrogene moxeparvovec-rokl is contraindicated in individuals with any deletion in exon 8 and/or exon 9 in the DMD gene due to the increased risk for a severe immune-mediated myositis reaction. Common adverse reactions (incidence ≥5%) were vomiting and nausea, liver injury, pyrexia, and thrombocytopenia. (1)

On June 24, 2025, FDA issued a safety communication acknowledging that it received two reports of fatal acute liver failure in non-ambulatory pediatric male patients with DMD following treatment with delandistrogene moxeparvovec-rokl. Both cases, reported from clinical trial and post marketing data, involved elevated transaminases and hospitalization within two months of treatment. (19) Subsequently on July 18, 2025, FDA placed Sarepta Therapeutics investigational gene therapy clinical trials for limb girdle muscular dystrophy on clinical hold following three deaths potentially related to these products and new safety concerns that the study participants are or would be exposed to an unreasonable and significant risk of illness or injury. The FDA has also revoked Sarepta's platform technology designation and requested Sarepta to voluntarily stop all shipments of Elevidys. (20) On July 28,

2025, FDA removed the voluntary hold for ambulatory patients to receive Elevidys following the conclusion of its investigation that the death of the 8-year-old boy was unrelated to the gene therapy product itself. (21)

Table 3. Summary of Pivotal Trials

Study	Study 102 (1) (NCT03769116)	Study 103 (1) (NCT04626674)	Study 301 (1) (NCT05096221)
Study Type	Part 1: DBRCT; placebo-controlled (48 weeks) Part 2: Cross-over, blinding maintained (48 weeks)	Open-label, single-arm	DBRCT
Country	US	US	Global
Sites	2	5	42
Dates	2018-2026	2018-2028	2021-2024
Participants	Inclusion • Male at birth ambulatory DMD individuals aged 4 through 7 years • Confirmed frameshift mutation, or a premature stop codon mutation between exons 18 to 58 in the *DMD* gene • Indication of symptomatic muscular dystrophy by protocol-specified criteria Exclusion • Impaired cardiovascular function on ECHO. • Exposure to another investigational drug or exon skipping medication within 6	Inclusion • Male at birth ambulatory DMD individuals aged 4 through 7 years (Cohort 1) • Has a definitive diagnosis of DMD based on documented clinical findings and prior genetic testing Exclusion • Exposure to gene therapy, investigational medication, or any treatment designed to increase dystrophin expression within protocol-specified time limits. • Abnormality in protocol-specified diagnostic evaluations or laboratory tests Primary endpoint	Inclusion • Male at birth ambulatory DMD individuals aged ≥4 to <8 years • Has a definitive diagnosis of DMD based on documented clinical findings and prior genetic testing[b] • Has a NSAA score > 16 and < 29 at the screening visit. • Has a time to rise from floor < 5 seconds at the screening visit Exclusion • Exposure to gene therapy, investigational medication, or any treatment designed to

	months of screening. • Abnormal liver or renal function by protocol-specified criteria Primary endpoint(s) • Change in the quantity of delandistrogene moxeparvovec-rokl micro-dystrophin protein from baseline to week 12 (Part 1), as measured by western blot • Change in NSAA total score from baseline to week 48 (Part 1)	• Change from baseline in quantity of delandistrogene moxeparvovec micro-dystrophin protein expression at week 12	increase dystrophin expression within protocol-specified time limits. • Abnormality in protocol-specified diagnostic evaluations or laboratory tests • Left ventricular ejection fraction < 40% on the screening ECHO or clinical signs and/or symptoms of cardiomyopathy Primary endpoint • Change in NSAA total score from baseline to Week 52.[c] Key secondary endpoints • Quantity of micro-dystrophin protein expression at week 12 as measured by western blot • Change in time to rise from the floor from baseline to week 52 • Change in time of 10-meter walk/run from

			baseline to week 52
Intervention			
Active	• Single intravenous infusion of delandistrogene moxeparvovec-rokl via a peripheral limb (n=20) Dosing[a] • 8 received intended dose (1.33 X 10^{14} vg/kg) • 6 received two-thirds of intended dose (8.94 X 10^{13} vg/kg) • 6 received half of the intended dose (6.29 X 10^{13} vg/kg)	• Cohorts 1 (n=20, ambulatory), 2 (n=7, ambulatory) and 3 (n=6, non-ambulatory): Confirmed frameshift, splice site or premature stop codon mutation anywhere in the *DMD* gene • Cohort 4 (n=7, ambulatory): Confirmed mutations in the *DMD* gene starting at or after exon 18. • Cohort 5a (n=6, ambulatory): Confirmed mutations that partially or fully overlap with exons 1-17 in the *DMD* gene. • Cohort 5b (n=2, non-ambulatory): Confirmed mutations that partially or fully overlap with exons 1-17 in the *DMD* gene. • Single intravenous infusion of delandistrogene moxeparvovec-rokl via a peripheral limb at the intended dose (1.33 X 10^{14} vg/kg) if they weighed less than 70 kg or 9.31 X 10^{15} vg/kg total fixed dose if they weighed 70 kg or greater. • N=48	• Single intravenous infusion of delandistrogene moxeparvovec-rokl via a peripheral limb at the intended dose (1.33 X 10^{14} vg/kg), n=63
Control	Placebo (N=21)	None	Placebo (n=62)
Follow-Up	48 weeks	12 weeks	52 weeks

DBRCT: double-blind randomized controlled trial; DMD: Duchenne muscular dystrophy; ECHO: echocardiogram; FDA: US Food and Drug Administration; NCT: national clinical trial identification number; NSAA: North Star Ambulatory Assessment.

[a] It is unclear if the variation in dosing was prespecified or not. According to the FDA documents, manufacturer retrospectively determined that in the delandistrogene moxeparvovec-rokl group, 12 of 20 participants received less than the intended dose This discrepancy was identified following a change in the analytical method for dose determination.

[b] Genetic report must describe a frameshift deletion, frameshift duplication, premature stop ("nonsense"), canonical splice site mutation, or other pathogenic variant in the DMD gene fully contained between exons 18 to 79 (inclusive) that is expected to lead to absence of dystrophin protein. The following mutations were not eligible: mutations between or including exons 1-17, in-frame deletions, in-frame duplications, and variants of uncertain significance and mutations fully contained within exon 45 (inclusive).

[c] Assuming a standard deviation of 3.5 in all subjects and a 10% dropout rate at week 52, with a type I error of 0.05 (2-sided), a sample size of 120 with 1:1 randomization ratio will provide approximately 90% power to detect a mean difference of 2.2 in change in NSAA total score from baseline to week 52 between the treated arm and placebo arm.

Table 4. Summary of Baseline Demographics and Disease Characteristics in the Pivotal Studies

	Study 102 (1)			**Study 301 (1)**		
	All (n=41)	**Delandistrogene moxeparvovec-rokl (n=20)**	**Placebo (n=21)**	**All (N=48)**	**Delandistrogene moxeparvovec-rokl (n=63)**	**Placebo (n=62)**
Race group White (%)	73	65	81	77	78	74
Mean age [range] (years)	6.27 (4.34 to 7.98)	6.29 (4.47 to 7.85)	6.24 (4.34 to 7.98)	7.7 (3.2 to 20.2)	6.0 (4.1 to 7.9)	6.1 (4.0 to 7.9)
Mean weight [range] (kg)	22.4 (15.0 to 34.5)	23.3 (18.0 to 34.5)	21.6 (15.0 to 30.0)	30.1 (12.5 to 80.1)	21.3 (13.5 to 38.5)	22.4 (14.4 to 41.6)
Mean NSAA total score [range]	21.2 (13 to 29)	19.8 (13 to 26)	22.6 (15 to 29)	20.3 (11 to 30)[a]	23.1 (14 to 32)	22.8 (15.5 to 30)
Mean time to rise from floor [range] (seconds)	4.3 (2.7 to 10.4)	5.1 (3.2 to 10.4)	3.6 (2.7 to 4.8)	4.7 (2.4 to 9.7)[a]	3.52 (1.9 to 5.8)	3.60 (2.3 to 5)

[a] NSAA and Time to rise from floor were not evaluated in non-ambulatory individuals.

NSAA: North Star Ambulatory Assessment.

Table 5. Summary of Clinical Outcomes in Pivotal Trials

Study 102 (1)	**Delandistrogene moxeparvovec-rokl**	**Placebo**	**P value**
Least square mean changes (±SE) in NSAA total score from baseline to week 48			
All ages	1.7 (0.6) (n=20)	0.9 (0.6) (n=21)	.37
Age group 4 through 5	4.3 (±0.7) (n=8)	1.9 (±0.7) (n=8)	Exploratory analysis
Age group 6 through 7	-0.2 (±0.7) (n=12)	0.5 (±0.7) (n=13)	Exploratory analysis
Study 301 (1)	**Delandistrogene moxeparvovec-rokl, Least square mean changes (95% CI) from baseline to week 52**	**Placebo, Least square mean changes (95% CI) from baseline to week 52**	**Least square mean difference (95% CI), P value**
NSAA total score	2.57 (1.80 to 3.34) (n=63)	1.92 (1.14 to 2.70) (n=61)	0.65 (-0.45 to 1.74), p=.24
Time to rise from the floor (seconds)	-0.27 (-0.56, 0.02) (n=63)	0.37 (0.08, 0.67) (n=61)	-0.64 (-1.06, -0.23), exploratory analysis
Time of 10-meter walk/run (seconds)	-0.34 (-0.55, -0.14) (n=63)	0.08 (-0.13, 0.29) (n=61)	-0.42 (-0.71, -0.13), exploratory analysis
Time of 100-meter walk/run (seconds)	-6.57 (-10.05, -3.09) (n=59)	-3.28 (-6.86, 0.29) (n=57)	-3.29 (-8.28, 1.70), exploratory analysis
Time to ascent 4 steps (seconds)	-0.44 (-0.69, -0.20) (n=62)	-0.08 (-0.33, 0.17) (n=60)	-0.36 (-0.71, -0.01), exploratory analysis

NSAA: North Star Ambulatory Assessment; SE: standard error.

Table 6. Summary of Results on Biomarker Data in Pivotal Studies

	Western blot (% of delandistrogene moxeparvovec-rokl micro-dystrophin compared to control) at week 12[a,b,c]
Study 102 (1)	
Mean change from baseline (±SD) in part 1 [d](n=6)	43.4% (± 48.6)
Median change from baseline (range) in part 1 [d](n=6)	24.3% (1.6, 116.3)

Mean change from baseline (±SD) in part 2 [e](n=21)	40.7% (± 32.3)
Median change from baseline (range) in part 2 [e](n=21)	40.8% (0.0, 92.0)
Study 103 (1)	
Mean change from baseline (±SD) among ambulatory (n=40)	51.0% (±47)
Median change from baseline (range) among ambulatory (n=40)	46.9% (1.9, 197.3)
Mean change from baseline (±SD) among non-ambulatory (n=8)	40.1% (±35.9)
Median change from baseline (range) among non-ambulatory (n=8)	32.7% (1.4, 116.3)

RCT: randomized controlled trial; SD: standard deviation.

[a] All individuals received 1.33 x 10^{14} vg/kg delandistrogene moxeparvovec-rokl, as measured by droplet digital polymerase chain reaction.

[b] Change from baseline was statistically significant.

[c] Adjusted for muscle content. Control was level of wild-type (normal) dystrophin in normal muscle.

[d] Part 1 of the study was the double-blind RCT phase for 48 weeks.

[e] Part 2 of the study was the cross-over phase where participants who received placebo in part 1 received now received delandistrogene moxeparvovec-rokl.

Table 7. Side by Side Comparison of Efficacy Results of Study 301 and 102[a]

Endpoint	**Analysis**	**Difference in LSM (95% CI) at week 52 in Study 301**	**Difference in LSM (95% CI) at week 48 in Study 102**	**Are results directionally consistent[b] between the 2 studies?**
Primary Endpoint: NSAA total score	Overall	0.65 (-0.45, 1.74)	0.82 (-1.03, 2.67)	Yes
	4-5 years old	1.32 (-0.23, 2.87)	2.47 (0.52, 4.43)	Yes
	6-7 years old	0.06 (-1.52, 1.64)	-0.70 (-3.02, 1.62)	No
Key Secondary Endpoint: Time to rise from floor (seconds)	Overall	-0.64 (-1.06, -0.23)	-0.50 (-1.22, 0.23)	Yes
	4-5 years old	-0.50 (-0.90, -0.09)	-0.30 (-1.32, 0.72)	Yes
	6-7 years old	-0.78 (-1.48, -0.08)	-0.56 (-1.59, 0.47)	Yes
Key Secondary Endpoint: 10-MWR timed test (seconds)	Overall	-0.42 (-0.71, -0.13)	0.49 (-0.08, 1.06)	No
	4-5 years old	-0.33 (-0.62, -0.03)	0.16 (-0.69, 1.02)	No

	6-7 years old	-0.52 (-1.01, -0.03)	0.76 (-0.01, 1.54)	No

[a] Reproduced from Table 9 in the FDA Statistical Review[8].
[b] Directionally consistent implies the point estimates were on the same side of null.
10-MWR: 10-meter walk/run; CI: confidence interval; LSM: least square mean; NSAA: North Star Ambulatory Assessment.

The purpose of the study limitations tables (Tables 8 and 9) is to display notable limitations identified in each study. This information is synthesized as a summary of the body of evidence and provides the conclusions on the sufficiency of evidence supporting the position statement. Multiple major limitations were noted. Both randomized, placebo-controlled studies failed to show a statistically significant difference in the pre-specified primary endpoint which was change in the NSAA total score between the treated and the placebo group. The US FDA approval was based on the post-hoc exploratory analysis of secondary outcome measures such as 10-MWR and time to rise from floor. These results cannot be interpreted at face value due to the lack of pre-specification and control of type 1 error. Such post hoc analysis following an overall nonsignificant test in the overall population can only be considered as hypothesis-generating. In addition, the observed treatment effect was not substantial and of uncertain clinical significance. The upper bound of 95 percent confidence intervals of point estimates for time to rise and 10-MWR was near the zero point (no effect). Further, the observed effect on 10-MWR timed test was also inconsistent with opposing results observed in the 2 RCTs. The decisional memorandum released by the Director of the Center for Biologics Evaluation and Research to clarify his action of overruling the staff's recommendation for a complete response letter referenced an exploratory study indicating a moderate correlation between micro-dystrophin levels and the 10-MWR as well as the time required to climb 4 steps, as supportive evidence. (11) However, it's important to note that micro-dystrophin expression levels were measured in only 25% of the study participants enrolled in study 301. Consequently, these findings might not accurately reflect the association between micro-dystrophin and the clinical efficacy outcomes across the full study cohort. Because of all these limitations in the current evidence, an adequately powered randomized, double-blind, placebo-controlled trial is necessary to clearly ascertain the net health outcome in DMD. Lastly, biomarker data reported in studies only provides information about expression of the transgene product in cells transduced by delandistrogene moxeparvovec-rokl rather than insight into a pharmacologic effect on a known biomarker in the pathway of the disease. Delandistrogene moxeparvovec-rokl micro-dystrophin is a novel, engineered protein that contains selected domains of the normal, wild-type dystrophin expressed in healthy muscle cells. No epidemiologic or pathophysiologic evidence is available regarding the function of delandistrogene moxeparvovec-rokl micro-dystrophin. The protein differs in important ways from both the endogenous shortened forms of dystrophin in individuals with Becker muscular dystrophy, and the internally truncated dystrophins expressed through exon-skipping drugs.

Table 8. Study Relevance Limitations

Study	Study 102 (1)	Study 103 (1)	Study 301 (1)

Population[a]	4. Enrolled populations do not reflect relevant diversity (73% White)	4. Enrolled populations do not reflect relevant diversity (75% White)	4. Enrolled populations do not reflect relevant diversity (78-74% White)
Intervention[b]			
Comparator[c]			
Outcomes[d]		1. Key health outcomes not addressed 2. Physiologic measures not validated surrogates (delandistrogene moxeparvovec-rokl microdystrophin is not an established surrogate biomarker) 5. Clinically significant difference not prespecified 6. Clinically significant difference not supported	
Duration of Follow-up[e]	1. Not sufficient duration for benefit 2. Not sufficient duration for harms	1. Not sufficient duration for benefit 2. Not sufficient duration for harms	1. Not sufficient duration for benefit 2. Not sufficient duration for harms

The study limitations stated in this table are those notable in the current review; this is not a comprehensive gaps assessment.

[a] Population key: 1. Intended use population unclear; 2. Study population is unclear; 3. Study population not representative of intended use; 4, Enrolled populations do not reflect relevant diversity; 5. Other.

[b] Intervention key: 1. Not clearly defined; 2. Version used unclear; 3. Delivery not similar intensity as comparator; 4. Not the intervention of interest (e.g., proposed as an adjunct but not tested as such); 5: Other.

[c] Comparator key: 1. Not clearly defined; 2. Not standard or optimal; 3. Delivery not similar intensity as intervention; 4. Not delivered effectively; 5. Other.

[d] Outcomes key: 1. Key health outcomes not addressed; 2. Physiologic measures, not validated surrogates; 3. Incomplete reporting of harms; 4. Not establish and validated measurements; 5. Clinically significant difference not prespecified; 6. Clinically significant difference not supported; 7. Other.

[e] Follow-Up key: 1. Not sufficient duration for benefit; 2. Not sufficient duration for harms; 3. Other.

Table 9. Study Design and Conduct Limitations

Study	Study 102 (1)	Study 103 (1)	Study 301 (1)

Allocation[a]		1. Participants not randomly allocated 2. Allocation not concealed 3. Allocation concealment unclear 4. Inadequate control for selection bias	
Blinding[b]		1. Participants or study staff not blinded 2. Outcome assessors not blinded 3. Outcome assessed by treating physician 4. Outcomes not assessed centrally	
Selective Reporting[c]			
Data Completeness[d]			
Power[e]		1. Power calculations not reported 2. Power not calculated for primary outcome 3. Power not based on clinically important difference	
Statistical[f]	5. Other (post-hoc sub-group analysis is exploratory)		5. Other (although the study was stratified based on age at baseline (≥4 to <6 years, or ≥6 to <8 years), age subgroup analyses were not prespecified for hypothesis testing,

			and no prespecified multiplicity adjustment strategy was employed. The study was not designed with prespecified analyses of any secondary endpoints for hypothesis testing, or with a prespecified multiplicity adjustment strategy. Consequently, one cannot reliably distinguish if any of those results are due to actual effects of delandistrogene moxeparvovec-rokl, or to chance alone.)

The study limitations stated in this table are those notable in the current review; this is not a comprehensive gaps assessment.

[a] Allocation key: 1. Participants not randomly allocated; 2. Allocation not concealed; 3. Allocation concealment unclear; 4. Inadequate control for selection bias; 5. Other.

[b] Blinding key: 1. Participants or study staff not blinded; 2. Outcome assessors not blinded; 3. Outcome assessed by treating physician; 4. Other.

[c] Selective Reporting key: 1. Not registered; 2. Evidence of selective reporting; 3. Evidence of selective publication; 4. Other.

[d] Data Completeness key: 1. High loss to follow-up or missing data; 2. Inadequate handling of missing data; 3. High number of crossovers; 4. Inadequate handling of crossovers; 5. Inappropriate exclusions; 6. Not intent to treat analysis (per protocol for noninferiority trials); 7. Other.

[e] Power key: 1. Power calculations not reported; 2. Power not calculated for primary outcome; 3. Power not based on clinically important difference; 4. Other.

[f] Statistical key: 1. Analysis is not appropriate for outcome type: (a) continuous; (b) binary; (c) time to event; 2. Analysis is not appropriate for multiple observations per patient; 3. Confidence intervals and/or p values not reported; 4. Comparative treatment effects not calculated; 5. Other.

Section Summary: Gene Therapy for Treatment of Duchenne Muscular Dystrophy

Evidence for the use of delandistrogene moxeparvovec-rokl for the treatment individuals with DMD includes 2 RCTs (studies 102 and 301) and 1 prospective cohort trial (study 103). In study 102, 41 study participants were randomized 1:1 to receive either delandistrogene

moxeparvovec-rokl (n=20) or placebo (n=21). In study 301, 125 study participants were randomized 1:1 to receive either delandistrogene moxeparvovec-rokl (n=63) or placebo (n=62). Both studies failed to show a statistically significant difference in the primary endpoint of change in the NSAA total score between the treated and the placebo group. In study 102, the LS mean change in the NSAA total score from baseline to week 48 was 1.7 points for the delandistrogene moxeparvovec-rokl group and 0.9 points for the placebo group (p=.37). In study 301, the LS mean change in the NSAA total score from baseline to week 52 was 2.57 points for the delandistrogene moxeparvovec-rokl group and 1.92 points for the placebo group (p=.24). Thus, clinical benefit was not demonstrated in the primary efficacy endpoint of NSAA total score from baseline in both studies. Multiple limitations were noted. The US FDA approval was based on the post-hoc exploratory analysis of secondary outcome measures such as 10-MWR and time to rise from floor. These results cannot be interpreted at face value due to the lack of pre-specification and control of type 1 error. Such post hoc analysis following an overall nonsignificant test in the overall population can only be considered as hypothesis-generating. In addition, the observed treatment effect on secondary outcomes was not substantial and of uncertain clinical significance. Further, the results of 10-MWR timed test were inconsistent with opposing results observed in the 2 RCTs. Because of these limitations, an adequately powered randomized, double-blind, placebo-controlled trial is necessary to clearly ascertain the net health outcome in DMD. Lastly, biomarker data reported in studies only provides information about expression of the transgene product in cells transduced by delandistrogene moxeparvovec-rokl rather than insight into a pharmacologic effect on a known biomarker in the pathway of the disease. Delandistrogene moxeparvovec-rokl micro-dystrophin is a novel, engineered protein that contains selected domains of the normal, wild-type dystrophin expressed in healthy muscle cells. No epidemiologic or pathophysiologic evidence is available regarding the function of delandistrogene moxeparvovec-rokl micro-dystrophin. The protein differs in important ways from both the endogenous shortened forms of dystrophin in individuals with Becker muscular dystrophy, and the internally truncated dystrophins expressed through exon-skipping drugs. Thus, the clinical benefit of treating DMD with delandistrogene moxeparvovec-rokl, including improved motor function and pulmonary function, has not been demonstrated. A confirmatory, prospective, and adequately powered trial is necessary to assess the net health outcome of delandistrogene moxeparvovec-rokl in individuals with DMD.

Ongoing and Unpublished Clinical Trials

Some currently ongoing trials that might influence this policy are listed in Table 10.

Table 10. Summary of Key Trials

NCT Number	Trial Name	Planned Enrollment	Completion Date
NCT04626674[a] (ENDEAVOR, Study 103)	A Gene Transfer Therapy Study to Evaluate the Safety of and Expression From SRP-9001 (Delandistrogene Moxeparvovec) in Participants With Duchenne Muscular Dystrophy	55	Jul 2026

NCT05881408[a] (ENVISION, Study 303)	A Gene Transfer Therapy Study to Evaluate the Safety and Efficacy of SRP-9001 (Delandistrogene Moxeparvovec) in Non-Ambulatory and Ambulatory Participants With Duchenne Muscular Dystrophy	148	Jun 2028
NCT06128564 (ENVOL)	A Gene Delivery Study to Evaluate the Safety and Expression of Delandistrogene Moxeparvovec in Participants Under the Age of Four With Duchenne Muscular Dystrophy	21	May 2033
NCT05967351 (EXPEDITION)	A Long-term Follow-up Study of Participants Who Received Delandistrogene Moxeparvovec (SRP-9001) in a Previous Clinical Study	400	Nov 2030
NCT06241950	A Gene Transfer Therapy Study to Evaluate the Safety and Efficacy of Delandistrogene Moxeparvovec (SRP-9001) Following Imlifidase Infusion in Participants With Duchenne Muscular Dystrophy Determined to Have Pre-existing Antibodies to Recombinant Adeno-Associated Virus Serotype	6	Oct 2027
NCT06270719 (ENDURE)	An Observational Study Comparing Delandistrogene Moxeparvovec With Standard of Care in Participants With Duchenne Muscular Dystrophy	500	Dec 2038

NCT: national clinical trial.

[a] Denotes industry-sponsored or cosponsored trial.

Coding

Procedure codes on Medical Policy documents are included **only** as a general reference tool for each policy. **They may not be all-inclusive.**

The presence or absence of procedure, service, supply, or device codes in a Medical Policy document has no relevance for determination of benefit coverage for members or reimbursement for providers. **Only the written coverage position in a Medical Policy should be used for such determinations.**

Benefit coverage determinations based on written Medical Policy coverage positions must include review of the member's benefit contract or Summary Plan Description (SPD) for defined coverage vs. non-coverage, benefit exclusions, and benefit limitations such as dollar or duration caps.

CPT Codes	None
HCPCS Codes	J1413

*Current Procedural Terminology (CPT®) ©2024 American Medical Association: Chicago, IL.

References

U.S. Food and Drug Administration Label

1. Prescribing Label: Elevidys (delandistrogene moxeparvovec-rokl) suspension, for intravenous infusion. Revised: 11/2025. Available at <https://www.fda.gov> (accessed on December 14, 2025).

Other

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Centers for Medicare and Medicaid Services (CMS)

The information contained in this section is for informational purposes only. HCSC makes no representation as to the accuracy of this information. It is not to be used for claims adjudication for HCSC Plans.

The Centers for Medicare and Medicaid Services (CMS) does not have a national Medicare coverage position. Coverage may be subject to local carrier discretion.

A national coverage position for Medicare may have been developed since this medical policy document was written. See Medicare's National Coverage at <https://www.cms.hhs.gov>.

Policy History/Revision	
Date	**Description of Change**
01/01/2026	Document updated. The following change was made to Coverage: Removed not medically necessary statement. Added references 6-21; other(s) updated.
07/01/2025	Document updated. The following change was made to Coverage: Added "Submitted genetic testing for DMD confirms the individual does not have a deletion in exon 8 and/or 9; AND" to conditional criteria. No new references added.
02/15/2025	Reviewed. No changes.
09/01/2024	Document updated with literature review. Coverage was revised to state Delandistrogene moxeparvovec-rokl (Elevidys) may be considered medically necessary when ALL the following criteria are met: Individuals ≥ 4 years of age; AND Individual has a diagnosis of Duchenne muscular dystrophy (DMD) confirmed by a mutation in the DMD gene; AND Individual is ambulatory. Delandistrogene moxeparvovec-rokl (Elevidys) for the treatment of Duchenne muscular dystrophy (DMD) in non-ambulatory individuals is considered not medically necessary as a clinical benefit has not been established. References revised; none added.
01/15/2024	New medical document. Delandistrogene moxeparvovec-rokl (Elevidys) for the treatment of Duchenne muscular dystrophy (DMD) is considered not medically necessary as a clinical benefit has not been established. Delandistrogene moxeparvovec-rokl (Elevidys) for the treatment of all other indications is considered experimental, investigational and/or unproven.