Pharmacy Drug Policy

SUBJECT: Elevidys (delandistrogene moxeparvovec-rokl)
POLICY NUMBER: PHARMACY-117
EFFECTIVE DATE: 04/2024
LAST REVIEW DATE: 11/18/2026

If the member's subscriber contract excludes coverage for a specific service or prescription drug, it is not covered under that contract. In such cases, medical or drug policy criteria are not applied. This drug policy applies to the following line/s of business:

Policy Application

Category:	☒ Commercial Group (e.g., EPO, HMO, POS, PPO)	☒ Medicare Advantage
	☒ On Exchange Qualified Health Plans (QHP)	☐ Medicare Part D
	☒ Off Exchange Direct Pay	☒ Essential Plan (EP)
	☒ Medicaid & Health and Recovery Plans (MMC/HARP)	☒ Child Health Plus (CHP)
	☐ Federal Employee Program (FEP)	☐ Ancillary Services
	☒ Dual Eligible Special Needs Plan (D-SNP)	

DESCRIPTION:

Elevidys (delandistrogene moxeparvovec-rokl) is an adeno-associated virus vector-based gene therapy indicated for the treatment of ambulatory pediatric patients aged 4 through 5 years of age. It is designed to deliver the gene encoding the micro-dystrophin protein. The micro-dystrophin expressed by Elevidys is a shortened version that contains selected domains of dystrophin expressed in normal muscle cells. The drug was initially granted accelerated approval based on increased expression of micro-dystrophin, a surrogate biomarker, in the absence of conclusive clinical efficacy data. The functional correlation of micro-dystrophin to native dystrophin remains under investigation.

The accelerated approval of Elevidys was based upon data from two ongoing small-scale clinical studies (Study 102 and Study 103) and safety data from three ongoing trials (Study 101, Study 102, and Study 103). Study 102 is a multicenter three-part Phase 2 study and Study 3 is a two-part open-label phase 1 study in five cohorts of boys with DMD defined by age and ambulatory status. For the subset of patients 4-5 years of age who received the FDA approved dosage of Elevidys, the mean change from baseline in Elevidys micro-dystrophin expression levels at Week 12 following Elevidys infusion was 95.7% (n=3; standard deviation [SD]: 17.9%) in Study 102 Parts 1 and 2, and 51.7% (n=11; SD: 41.0%) in Study 103 Cohort 1. Elevidys did not demonstrate a statistically significant treatment effect on functional outcomes; however, an exploratory subgroup analysis of the 16 participants (Elevidys: n=8; placebo: n=8) 4 through 5 years of age showed a numerical advantage for Elevidys compared to placebo in the change in North Star Ambulatory Assessment (NSAA) total score.

On June 21, 2024, The FDA has converted the accelerated approval to Elevidys (delandistrogene moxeparvovec-rokl) to a full approval to treat ambulatory patients with Duchenne muscular dystrophy aged 4 years and older. The agency also granted accelerated approval to Elevidys for non-ambulatory patients.

The Phase 3 EMBARK study is being conducted as the confirmatory trial for Elevidys to assess clinical benefit. On October 30, 2023, Sarepta announced topline results from EMBARK, which enrolled 125 patients with DMD between the ages of 4-7 years of age. The primary endpoint was not met as the change in NSAA total score from baseline at Week 52 (2.6 points in Elevidys-treated patients vs 1.9 points in placebo-treated) did not reach statistical significance (n=125, p=0.24). Key secondary endpoints, including Time to rise (TTR) and 10-meter walk test, showed statistically significant

improvement (Change vs placebo LSM difference in seconds was -0.64 for TTR and -0.42 for 10-meter walk test).

Elevidys is contraindicated in patients with any deletion in exon 8 and/or exon 9 in the DMD gene due to risk for immune-mediated myositis. Warnings/precautions involve acute serious liver injury, immune-mediated myositis, myocarditis, and pre-existing immunity against AAVrh74.

On June 15, 2025, Sarepta announced suspension of commercial shipments of Elevidys for non-ambulatory patients following two treatment-related deaths due to acute liver failure. As a result, the FDA added a Boxed Warning for acute serious liver injury and acute liver failure, and the non-ambulatory indication was removed from Elevidys' label.

Two systematic reviews and meta-analyses were conducted in 2025 and 2026. In the 2025 analysis, compared with placebo, the only pooled improvement reported was supine-to-stand time (−0.64 seconds; 95% CI −0.99 to −0.30). Effects on some outcomes tended to be greater in participants aged 4–5 years. Authors concluded at 1 year after treatment, Elevidys had a modest effect on DMD progression, and the authors state that longer trials are needed to confirm efficacy. The pooled NSAA gain comes from uncontrolled pre-post data, and the only placebo-controlled benefit (supine-to-stand time) closely mirrors EMBARK's Time to Rise result, a secondary endpoint for which no statistical significance can be claimed. In the 2026 analysis, nonrandomized studies generally reported larger treatment effects. Randomized, placebo-controlled trials showed smaller or nonsignificant effects. Authors concluded the true effect is likely modest. Heterogeneity was extreme ($I^2 \approx 99\%$), and the pooled estimates depend heavily on nonrandomized data.

POLICY:

ELEVIDYS (DELANDISTROGENE MOXEPARVOVEC-ROKL)

Commercial/Essential/Child Health Plus Policy:

Based on a review of the available peer-reviewed evidence, delandistrogene moxeparvovec (Elevidys) is considered not medically necessary for the treatment of Duchenne muscular dystrophy (DMD).

The basis for accelerated approval was the surrogate marker of micro-dystrophin expression, which was confirmed (34.29% in the treatment group vs 0.00% in placebo at Week 12). However, there is insufficient evidence to conclude that this biologic effect translates into statistically significant or clinically meaningful motor-function benefit. As a condition of approval, the FDA is requiring Sarepta Therapeutics to complete a clinical study to confirm Elevidys' clinical benefit. Thus, Sarepta conducted the global EMBARK trial.

In the 52-week, randomized, double-blind, placebo-controlled, phase 3 "EMBARK" trial (NCT05096221), which enrolled 125 ambulatory boys 4 to <8 years of age with a confirmed DMD mutation, delandistrogene moxeparvovec did not meet its primary endpoint. The change from baseline to Week 52 in North Star Ambulatory Assessment (NSAA) total score was 2.57 points (least-squares mean) in the treatment group compared to 1.92 points in the placebo group, a between-group difference of 0.65 points (95% CI −0.45 to 1.74; p=0.24), which did not reach statistical significance. Key functional secondary endpoints, including Time to Rise (TTR) and the 10-meter walk/run test (10MWR), numerically favored treatment (LSM difference −0.64 seconds for TTR and −0.42 seconds for 10MWR). However, because the primary endpoint failed, these secondary endpoints were not met within the prespecified hierarchical testing framework and no statistical significance can be claimed.

The confirmatory randomized comparison (EMBARK) did not demonstrate a statistically significant difference on its primary clinical endpoint.

Additional clinical data beyond the 52-week randomized period are limited and do not resolve this uncertainty. Because patients in the placebo arm switched to active treatment after Week 52, longer-term outcomes were compared with matched patients from outside the study rather than with an ongoing randomized placebo group. The EMBARK 2-year analysis reported statistically significant differences versus an external control on NSAA, TTR, and 10MWR compared with external controls, along with sustained micro-dystrophin expression. Similarly, 5-year follow-up from phase 1/2a data (n=4) suggested continued ambulation versus external control. However, the American Academy of Neurology graded these external-control analyses as Class III evidence and noted they did not account for differences in dose and duration of concomitant corticosteroid exposure, an independent and important confounder of strength and timed-function outcomes in DMD. These datapoints were therefore not established as both statistically and clinically significant estimates of treatment-attributable benefit.

A 2026 systematic review and meta-analysis was conducted to evaluate both efficacy and safety of AAV-based mini- and micro-dystrophin therapies in DMD. Pooled data show modest functional gains measured by the North Star Ambulatory Assessment score, particularly in younger ambulatory patients, along with improvements in select timed function tests. Safety data are summarized across trials, highlighting mostly mild or transient adverse events; however, rare serious and fatal outcomes were also reported. Authors concluded findings support cautious clinical implementation of AAV-based gene therapies, particularly in younger patients, while emphasizing the need for careful patient selection and rigorous safety monitoring.

DMD is a rare, progressive, and heavily burdensome disease associated with loss of ambulation, respiratory and cardiac decline, and shortened life expectancy. While delandistrogene moxeparvovec produced measurable micro-dystrophin expression and numerically favorable functional trends, uncertainty remains regarding whether these changes translate into clinically meaningful improvements in patient outcomes. The only randomized, placebo-controlled (Class I) comparison failed its prespecified primary endpoint, and every supportive long-term dataset depends on non-randomized external controls that do not adjust for key confounders. Therefore, disease rarity, first-in-indication status, and unmet need were not considered sufficient to resolve the uncertainty surrounding the clinical impact of the observed effect.

Reconsideration of Medical Necessity

This determination will be reconsidered if new evidence addresses the uncertainty regarding the clinical and statistical significance of delandistrogene moxeparvovec (Elevidys) treatment-attributable benefit.

Evidence that may support reconsideration includes:

- A prespecified, statistically controlled analysis demonstrating a statistically significant and clinically meaningful incremental benefit on a validated motor-function endpoint (NSAA) versus placebo or an appropriate active comparator;
- Replication or confirmation of clinically meaningful functional benefit in an additional adequately designed, randomized, controlled study;
- Demonstration of sustained benefit beyond the currently established 52-week controlled treatment period using an appropriately controlled design rather than non-randomized external comparators;

- Statistically and clinically meaningful between-group improvement in patient-centered outcomes such as delay in loss of ambulation, preservation of pulmonary or cardiac function, or reduction in disease-related morbidity and mortality;
- Longer-term evidence that more broadly characterizes the benefit-risk profile, including durability of effect and the serious adverse events associated with this therapy (acute liver injury, immune-mediated myositis, myocarditis);
- Adequate adjustment for concomitant corticosteroid exposure and other prognostic confounders in any comparative analysis;
- Results from confirmatory studies verifying that micro-dystrophin expression translates into clinically meaningful functional benefit.

MMC/HARP Coverage Criteria:

1. Must be prescribed by or in consultation with a provider who specializes in the treatment of Duchene Muscular Dystrophy (DMD) **AND**
2. Must be at least 4 years old at time of treatment **AND**
3. Must have a diagnosis of DMD with a confirmed mutation in the DMD gene between exons 18-58
   a. Must NOT have a deletion in exon 8 and/or 9 in the DMD gene
   b. Must NOT have a mutation fully contained within exon 45 **AND**
4. Must be ambulatory as confirmed by the NSAA scale (patient score of 1 or greater) **AND**
5. Baseline anti-AAVrh74 total binding antibodies must be <1:400, as measure by ELISA **AND**
6. Must not have clinical signs or symptoms of infection at the time of Elevidys administration **AND**
7. Must be on a stable dose of corticosteroids for at least 3 months prior to therapy or a documented reason not to be on corticosteroids **AND**
8. Elevidys will not be authorized for use in combination with any other exon-skipping therapies for DMD (Exondys 51, Vyondys 53, Amondys 45, Viltepso) **AND**
9. Elevidys is indicated for one-time single-dose intravenous use only and therefore will not be authorized for retreatment. Retreatment will be considered Experimental/Investigational when any FDA approved gene therapy, or any other gene therapy under investigation, has been previously administered
10. Approval will be provided for 3 months to allow for the administration of the one-time treatment.

Medicare Advantage Coverage Criteria:

Medicare reviews are to follow the Local Coverage Determination (LCD) for Drugs and Biologicals, Coverage of, for Label and Off-Label Uses (L33394). The LCD can be found on the CMS website at: https://www.cms.gov/medicare-coverage-database/view/lcd.aspx?lcdid=33394

POLICY GUIDELINES:

1. Utilization Management is contract dependent. Refer to specific contract/benefit language for exclusions.
   a. Coverage criteria may be dependent on the contract renewal date.
   b. Coverage of drugs listed in this policy are contract dependent.
   c. Not all contracts/benefits allow coverage of healthcare professional administered drugs as part of their pharmacy benefit
   d. Not all contracts/benefits cover all medical infusible drugs.
   e. Not all policy criteria may apply if treatment of specific disease states or conditions is not a covered benefit.

2. Clinical documentation must be submitted for each request (initial and recertification [if applicable]) unless otherwise specified (e.g., provider attestation required). Supporting documentation includes, but is not limited to, progress notes documenting previous treatments and treatment history, diagnostic testing, laboratory test results, genetic testing or biomarker results, and imaging and other objective or subjective measure of clinical benefit.
3. Elevidys is administered intravenously and will be considered for coverage under the medical benefit.
4. This policy does not apply to Medicare Part D and D-SNP pharmacy benefits. The drugs in this policy may apply to all other lines of business including Medicare Advantage.
5. For members with Medicare Advantage, medications with a National Coverage Determination (NCD) and/or Local Coverage Determination (LCD) will be covered pursuant to the criteria outlined by the NCD and/or LCD. NCDs/LCDs for applicable medications can be found on the CMS website at https://www.cms.gov/medicare-coverage-database/search.aspx. In the absence of a Medicare National Coverage Determination (NCD), Local Coverage Determination (LCD), or other Medicare coverage guidance, CMS permits a Medicare Advantage Organization (MAO) to establish its own coverage determinations in accordance with 42 CFR § 422.101(b)(6). Indications that have not been addressed by the applicable medication's LCD/NCD will be covered in accordance with criteria determined by the Health Plan. Step therapy requirements may be imposed in addition to LCD/NCD requirements
6. Natural history data show that NSAA scores in corticosteroid-treated boys with DMD typically peak around 6 to 7 years and decline thereafter. Functional gains or stabilization observed in patients younger than 8 years may therefore reflect expected developmental patterns rather than therapeutic effect. Long-term preservation of function beyond age 8, including delayed loss of ambulation, is considered a clinically meaningful benchmark of disease-modifying therapy. Follow-up data beyond age 8 are now available (Study 101 five-year outcomes, n=4, EMBARK 3-year company topline results). However, these data rely on propensity-weighted external controls, do not account for differences in corticosteroid exposure, and in the case of Study 101 involve a very small sample treated with product from an earlier manufacturing process. As such, the Commercial/Essential/Child Health Plus policy will be re-reviewed upon availability of long-term data demonstrating sustained functional benefit in patients beyond the age of 8.
7. Manufacturers may either discontinue participation in, or may not participate in, the Medicaid Drug Rebate Program (MDRP). Under New York State Medicaid requirements, physician-administered drugs must be produced by manufacturers that participate in the MDRP. Products made by manufacturers that do not participate in the MDRP will not be covered under Medicaid Managed Care/HARP lines of business. Drug coverage will not be available for any product from a non-participating manufacturer. For a complete list of New/Reinstated & Terminated Labelers please visit: https://www.medicaid.gov/medicaid/prescriptiondrugs/medicaid-drug-rebate-program/newreinstated-terminated-labeler-information/index.html
8. All utilization management requirements outlined in this policy are compliant with applicable New York State insurance laws and regulations. Policies will be reviewed and updated as necessary to ensure ongoing compliance with all state and federally mandated coverage requirements.
9. This policy is based on available evidence as of the last review date. Coverage determinations are subject to applicable plan documents, state and federal regulations, and individual patient circumstances. This policy does not constitute medical advice.
10. For commercial contracts, medical necessity determinations align with the Certificate of Coverage issued by the Health Plan, which states that covered services must be clinically appropriate and not primarily for the convenience of the member, the member's family, or the provider.
11. This policy is subject to ongoing revision. Newly marketed drugs and existing drugs with new indications may be subject to prior authorization until formal coverage criteria are established. Inclusion of a drug in this policy does not guarantee its current availability on the market, as some

agents may be discontinued, withdrawn, or otherwise unavailable. As product status changes, drugs may be removed from the policy.

12. The requested site of care may impact approval timeframe and is subject to review.
13. The following applies to all gene and cellular therapies unless otherwise specified within the drug-specific coverage criteria:
    a. Administration, Retreatment, and Treatment with Additional or Other Gene/Cellular Therapies
        i. One-Time Administration
            1. Most gene and cellular therapies, whether autologous, allogeneic ("off-the-shelf"), or in vivo gene-transfer therapies, are designed and studied as one-time treatments.
            2. Repeat dosing, reinfusion, or sequential therapy with other gene or cellular products has not been established as safe, effective, or clinically appropriate.
        ii. Retreatment/Repeat Administration
            1. Retreatment with the same gene or cellular therapy product is considered experimental and investigational because:
                a. Clinical trials evaluated these therapies as single-administration interventions
                b. Safety, efficacy, and durability of a second administration have not been established
                c. Risk of immune activation, insertional mutagenesis, or vector immunity may be increased with repeat dosing
        iii. Treatment with an Additional or Other Gene/Cellular Therapy
            1. Treatment with an additional or different gene or cellular therapy after prior exposure to any gene or cellular therapy is currently considered experimental and investigational as there is lack of evidence demonstrating the following (a-c):
                a. Anticipated clinical benefit beyond available standard therapies
                b. Safety of sequential administration
                c. Justification for selecting a second gene/cellular intervention after a prior one
            2. This includes, but is not limited to:
                a. Switching between CAR-T products (e.g., CD19 → CD19 or CD19 → BCMA)
                b. Switching between autologous and allogeneic cellular therapies
                c. Sequential use of CAR-T, TCR-T, NK-cell therapies, or other genetically engineered cell therapies
                d. Receiving a gene therapy after previous gene or cellular therapy exposure
                e. Receiving an in vivo gene therapy following any prior vector-based therapy
        iv. Prior Gene/Cell Therapy Exposure
            1. An individual is generally not eligible for additional gene or cellular therapy if they have previously received:
                a. Any autologous cellular therapy (e.g., CAR-T, TCR-T, TIL),
                b. Any allogeneic genetically modified cellular therapy,
                c. Any in vivo gene therapy (e.g., AAV, lentiviral vector)
                d. Any ex vivo gene-modified cell product
                e. Are being considered for any other gene or cellular therapy without documented evidence supporting safety and anticipated benefit.
14. Approval timeframes may be extended to align authorization periods for drugs administered as part of a multidrug regimen, including when regimen components are administered across different benefits. This provision does not apply when the approval timeframe reflects a drug-specific limitation on duration of therapy.

CODES:

Eligibility for reimbursement is based upon the benefits set forth in the member's subscriber contract. CODES MAY NOT BE COVERED UNDER ALL CIRCUMSTANCES. PLEASE READ THE POLICY AND GUIDELINES STATEMENTS CAREFULLY.
Codes may not be all inclusive as the AMA and CMS code updates may occur more frequently than policy updates.

HCPCS:

J1413 Elevidys (Effective 1/1/2024)

UPDATES:

Date	Revision
11/12/2026	Revised. P&T Committee Review & Approval
09/14/2026	Revised
06/12/2026	Revised
01/30/2026	Revised
11/19/2025	Revised
11/13/2025	P&T Committee Review & Approval
06/24/2025	Revised
03/06/2025	Revised
01/31/2025	Revised
11/21/2024	P&T Committee Review & Approval
09/04/2024	Revised
06/20/2024	Revised
04/01/2024	Created & Implemented
11/23/2023	P&T Committee Review & Approval

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