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Section:	Prescription Drugs	**Effective Date:**	April 1, 2026
Subsection:	Neuromuscular Drugs	**Original Policy Date:**	August 18, 2023
Subject:	Elevidys	**Page:**	1 of 5

Last Review Date: March 6, 2026

Elevidys

Description

Elevidys (delandistrogene moxeparvovec-rokl)

Background
Elevidys (delandistrogene moxeparvovec-rokl) is the recombinant gene therapy product that is comprised of a non-replicating, recombinant, adeno-associated virus (AAV) serotype rh74 (AAVrh74) capsid and a ssDNA expression cassette flanked by inverted terminal repeats (ITRs) derived from AAV2. The cassette contains: 1) an MHCK7 gene regulatory component comprising a creatine kinase 7 promoter and an α-myosin heavy chain enhancer, and 2) the DNA transgene encoding the engineered Elevidys micro-dystrophin protein (1).

Vector/Capsid: Clinical and nonclinical studies have demonstrated AAVrh74 serotype transduction in skeletal muscle cells. Additionally, in nonclinical studies, AAVrh74 serotype transduction has been demonstrated in cardiac and diaphragm muscle cells (1).

Promoter: The MHCK7 promoter/enhancer drives transgene expression and has been shown in animal models to drive transgenic Elevidys micro-dystrophin protein expression predominantly in skeletal muscle (including diaphragm) and cardiac muscle. In clinical studies, muscle biopsy analyses have confirmed Elevidys micro-dystrophin expression in skeletal muscle (1).

Transgene: DMD is caused by a mutation in the *DMD* gene resulting in lack of functional dystrophin protein. Elevidys carries a transgene encoding a micro-dystrophin protein consisting of selected domains of dystrophin expressed in normal muscle cells (1).

Elevidys micro-dystrophin has been demonstrated to localize to the sarcolemma (1).

Regulatory Status
FDA-approved indication: Elevidys is an adeno-associated virus vector-based gene therapy indicated for the treatment of patients 4 years of age and older with Duchenne muscular dystrophy (DMD) who are ambulatory and have a confirmed mutation in the *DMD* gene (1).

Limitations of use: (1)
Elevidys is not recommended in patients with:

- Preexisting liver impairment (defined as gamma-glutamyl transferase [GGT] >2 x upper limit of normal or total bilirubin > the upper limit of normal not due to Gilbert's syndrome) or active hepatic viral infection due to the high risk of acute serious liver injury and acute liver failure.
- Recent vaccination (within 4 weeks of treatment) due to immunogenicity and potential safety concerns.
- Active or recent (within 4 weeks) infection due to safety concerns.

Elevidys carries a boxed warning for acute serious liver injury and acute liver failure. Patients with preexisting liver impairment may be at higher risk. Monitor liver function before Elevidys infusion, and weekly for the first 3 months after infusion. Continue monitoring until results are unremarkable. Patients should maintain proximity to an appropriate healthcare facility, as determined by the healthcare provider. If acute serious liver injury or impending acute liver failure is suspected, a consultation with a specialist is recommended (1).

Elevidys is contraindicated in patients with any deletion in exon 8 and/or exon 9, including a deletion of any portion or the entirety of these exons, in the *DMD* gene (1).

Patients with deletions in the *DMD* gene in exons 1 to 17 and/or exons 59 to 71 may be at risk for severe immune-mediated myositis reaction. Consider additional immunomodulatory treatment if symptoms of myositis occur (1).

Myocarditis and troponin-I elevations have been observed with Elevidys use. Monitor troponin-I before Elevidys infusion, and weekly for the first month after infusion (1).

Pre-existing immunity against AAVrh74 may occur. Perform baseline testing for presence of anti-AAVrh74 total binding antibodies prior to Elevidys administration (1).

The safety and effectiveness of Elevidys in patients less than 4 years of age have not been established (1).

Related policies
Agamree, Amondys 45, Duvyzat, Emflaza, Exondys 51, Viltepso, Vyondys 53

Policy

This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.

Elevidys may be considered **medically necessary** if the conditions indicated below are met.

Elevidys may be considered **investigational** for all other indications.

Prior-Approval Requirements

Age 4 years of age or older at the time of infusion

Gender assigned at birth Male

Diagnosis

Patient must have the following:

Duchenne muscular dystrophy (DMD)

AND ALL the following:

1. Genetic confirmation of DMD
2. **NO** deletion in exons 1 to 17 and/or exons 59 to 71 in the *DMD* gene
3. Anti-AAVrh74 total binding antibody titers are <1:400
4. Patient is ambulatory
5. Prescriber agrees that patient will **NOT** be using concomitant anti-sense oligonucleotides post-administration
6. Prescriber agrees to assess liver function prior to initiation, weekly for the first 3 months after infusion, and until results are unremarkable
7. Prescribed by or in consultation with a neurologist specializing in DMD
8. Patient has not previously received gene therapy for DMD
9. **NO** preexisting liver impairment (defined as gamma-glutamyl transferase [GGT] >2 x upper limit of normal or total bilirubin > the upper limit of normal not due to Gilbert's syndrome)

10. **NO** vaccination within 4 weeks of treatment
11. **NO** active or recent infection within 4 weeks

Prior – Approval *Renewal* Requirements

None

Policy Guidelines

Pre - PA Allowance

None

Prior - Approval Limits

Quantity One infusion (only one PA approval for one infusion per lifetime)

Prior – Approval *Renewal* Limits

None

Rationale

Summary

Elevidys (delandistrogene moxeparvovec-rokl) is an adeno-associated virus vector-based gene therapy indicated for the treatment of ambulatory patients 4 years of age and older with Duchenne muscular dystrophy (DMD) who are ambulatory with a confirmed mutation in the *DMD* gene. Elevidys carries a boxed warning regarding acute serious liver injury and acute liver failure. Elevidys is contraindicated in patients with any deletion in exon 8 and/or exon 9, including a deletion of any portion or the entirety of these exons, in the *DMD* gene. Myocarditis have been observed with Elevidys use. Immune-mediated myositis and pre-existing immunity against AAVrh74 may occur. The safety and effectiveness of Elevidys in patients less than 4 years of age have not been established (1).

Prior approval is required to ensure the safe, clinically appropriate, and cost-effective use of Elevidys while maintaining optimal therapeutic outcomes.

References

1. Elevidys [package insert]. Cambridge, MA: Sarepta Therapeutics, Inc; November 2025.

Policy History

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Date	Action
July 2023	Addition to PA
December 2023	Annual review
March 2024	Annual review
June 2024	Annual review and reference update
September 2024	Annual editorial review and reference update. Per SME, changed initiation requirement wording to “genetic confirmation of DMD”
December 2024	Annual review and reference update. Per association, expanded age requirement to 4 years of age or older at the time of infusion, removed the ambulatory requirement, and expanded exon deletion exclusions in criteria
March 2025	Annual review
December 2025	Per PI update, patient must be ambulatory, added boxed warning of acute serious liver injury and liver failure, added exclusion of preexisting liver impairment, recent vaccination, and active or recent infection
March 2026	Annual review
Keywords	

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on March 6, 2026 and is effective on April 1, 2026.