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Section:	Prescription Drugs	**Effective Date:**	January 1, 2024
Subsection:	Neuromuscular Drugs	**Original Policy Date:**	August 18, 2023
Subject:	Elevidys	**Page:**	1 of 4

Last Review Date: December 8, 2023

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 ambulatory pediatric patients aged 4 through 5 years with Duchenne muscular dystrophy (DMD) with a confirmed mutation in the *DMD* gene (1).

Elevidys is contraindicated in patients with any deletion in exon 8 and/or exon 9 in the *DMD* gene (1).

Acute serious liver injury has been observed with Elevidys use. Monitor liver function before Elevidys infusion, and weekly for the first 3 months after infusion. Continue monitoring until results are unremarkable. If acute serious liver injury is suspected, a consultation with a specialist is recommended (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 and greater than 5 years of age have not been established (1).

Related policies
Amondys 45, 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 through 5 years of age

Gender assigned at birth Male

Diagnosis

Patient must have the following:

Duchenne muscular dystrophy (DMD)

AND ALL the following:

1. Confirmed mutation of the *DMD* gene
2. **NO** deletion in exon 8 and/or exon 9 in the *DMD* gene
3. Patient is ambulatory
4. Anti-AAVrh74 total binding antibody titers are <1:400
5. Prescriber agrees that patient will **NOT** be using concomitant anti-sense oligonucleotides post-administration
6. Prescribed by or in consultation with a neurologist specializing in DMD
7. Patient has not previously received gene therapy for DMD

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 pediatric patients aged 4 through 5 years with Duchenne muscular dystrophy (DMD) with a confirmed mutation in the *DMD* gene. Elevidys is contraindicated in patients with any deletion in exon 8 and/or exon 9 in the *DMD* gene. Acute serious liver injury and 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 and greater than 5 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; July 2023.

Policy History

Date	Action
July 2023	Addition to PA
December 2023	Annual review

Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on December 8, 2023 and is effective on January 1, 2024.