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5.75.034

Section:	Prescription Drugs	**Effective Date:**	October 1, 2024
Subsection:	Neuromuscular Drugs	**Original Policy Date:**	September 18, 2020
Subject:	Viltepso	**Page:**	1 of 6

Last Review Date: September 6, 2024

Viltepso

Description

Viltepso (viltolarsen)

Background
Viltepso (viltolarsen) is an antisense oligonucleotide designed to bind to exon 53 on dystrophin pre-mRNA resulting in exclusion of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 53 skipping. Exon 53 skipping is intended to allow for production of an internally truncated dystrophin protein in patients with genetic mutations that are amenable to exon 53 skipping (1).

Regulatory Status
FDA-approved indication: Viltepso is an antisense oligonucleotide indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the DMD gene that is amenable to exon 53 skipping (1).

Renal toxicity, including potentially fatal glomerulonephritis, has been observed after administration of some antisense oligonucleotides. Serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio should be measured before starting Viltepso. Glomerular filtration rate using an exogenous filtration marker may also be measured before starting Viltepso. During treatment, urine dipstick should be monitored every month, and serum cystatin C and urine protein-to-creatinine ratio should be monitored every three months (1).

Monitoring motor changes in patients with DMD requires functional evaluation along with measurement of muscle strength. The need for a reliable outcome measure in diseases of rapid

deterioration such as DMD has led to the use of motor functional tests. In a large, multicenter, international clinical trial, the six minute walk test (6MWT) proved to be feasible and highly reliable. Also used are the Motor Function Measure (MFM) and North Star Ambulatory Assessment (NSAA) to help predict loss of ambulation 1 year before its occurrence in order to allow time to adapt rehabilitation, change the patient's environment, and consider acquisition of assistive aids or the use of medications (2-4).

Viltepso is indicated for patients who have a confirmed mutation of the DMD gene that is amenable to exon 53 skipping, including pediatric patients. DMD is largely a disease of children and young adults (1).

Related policies
Agamree, Amondys 45, Duvyzat, Elevidys, Emflaza, Exondys 51, 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.

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

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

Prior-Approval Requirements

Age 20 years of age or younger

Diagnosis

Patient must have the following:

Duchenne muscular dystrophy (DMD)

AND ALL the following:

1. Confirmed mutation of the DMD gene that is amenable to exon 53 skipping
2. Prescribed by or in consultation with a neurologist specializing in DMD

3. Prescriber agrees to monitor serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio for signs of kidney toxicity
4. Obtain a baseline muscle strength score from **ONE** of the following:
   a. 6-minute walk test (6MWT)
   b. North Star ambulatory assessment (NSAA)
   c. Motor Function Measure (MFM)
5. **NO** concurrent therapy with another exon skipping therapy for DMD (see Appendix 1)

Prior – Approval *Renewal* Requirements

Age 20 years of age or younger

Diagnosis

Patient must have the following:

Duchenne muscular dystrophy (DMD)

AND ALL of the following:

1. Prescriber agrees to monitor serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio for signs of kidney toxicity
2. Patient has had an improvement from baseline in **ONE** of the following:
   a. 6-minute walk test (6MWT)
   b. North Star ambulatory assessment (NSAA)
   c. Motor Function Measure (MFM)
3. **NO** concurrent therapy with another exon skipping therapy for DMD (see Appendix 1)

Policy Guidelines

Pre - PA Allowance

None

Prior - Approval Limits

Duration 12 months

Prior – Approval *Renewal* Limits

Duration 24 months

Rationale

Summary
Viltepso (viltolarsen) is an antisense oligonucleotide indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the DMD gene that is amenable to exon 53 skipping. Renal toxicity, including potentially fatal glomerulonephritis, has been observed after administration of some antisense oligonucleotides. DMD is largely a disease of children and young adults (1).

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

References

1. Viltepso [Package Insert]. Paramus, NJ: NS Pharma, Inc.; March 2021.
2. Mcdonald C, Henricson E, et al. The 6-Minute Walk test and Other Clinical Endpoints in Duchenne Muscular Dystrophy: Reliability, Concurrent Validity, and Minimal Clinically Important Differences from a Multicenter Study. Muscle Nerve. 2013 Sep; 48(3): 357‑368.
3. Mcdonald C, Henricson E, et al. The 6-Minute Walk test and Other Endpoints in Duchenne Muscular Dystrophy: Longitudinal Natural History Observations Over 48 weeks from a Multicenter Study. Muscle Nerve. 2013 Sep; 48(3): 343‑356.
4. Vuillerot C, Girardot F, et al. Monitoring changes and predicting loss of ambulation in Duchenne muscular dystrophy with the Motor Function Measure. *Developmental Medicine & Child Neurology* 2010, 52: 60–65.

Policy History

Date	Action
September 2020	Addition to PA
December 2020	Annual review. Per FEP, addition of requirement of no concurrent therapy with another exon skipping therapy for DMD
June 2021	Annual editorial review and reference update. Updated Appendix 1
December 2022	Annual review. Changed policy number to 5.75.034
December 2023	Annual review
June 2024	Annual review

September 2024	Annual editorial review

Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on September 6, 2024 and is effective on October 1, 2024.

Appendix 1 - List of Exon Skipping Therapies for Duchenne Muscular Dystrophy (DMD)

Generic Name	Brand Name
casimersen	Amondys 45
eteplirsen	Exondys 51
golodirsen	Vyondys 53
viltolarsen	Viltepso