Aetna Better Health® of New Jersey

Protocol for Duchenne Muscular Dystrophy Products
Updated October 2021

Approved July 2020
Updated July 2021 - Added viltolarsen (Viltepso®) – FDA-approved in August 2020

Exondys 51 (eteplirsen)
Vyondys 53 (golodirsen)
Viltepso (viltolarsen)
Amondys 45 (casimersen)

Addendum:
- Added casimersen (Amondys 45) - FDA-approved in February 2021
- Changed name to "Protocol for Duchenne Muscular Dystrophy Products"

Background:
- *Eteplirsen (Exondys 51)® 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 51 skipping.*
- *Golodirsen (Vyondys 53®) 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.*
- *Viltolarsen (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.*
- *Casimersen (Amondys 45®) 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 45 skipping*
  - *Limitations: This indication was approved under accelerated approval based on an increase in dystrophin production in skeletal muscle observed in patients treated with Vyondys 53/Viltepso/Amondys 45. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.*

Criteria for Approval:
1. Patient must have the diagnosis of Duchenne Muscular Dystrophy (DMD).
2. Submission of medical records including the following:
   a. For Exondys 51: Genetic testing confirming the patient has a mutation of the DMD gene that is amenable to exon 51 skipping
   b. For Vyondys 53 and Viltepso: Genetic testing confirming the patient has a mutation of the DMD gene that is amenable to exon 53 skipping.
   c. For Amondys 45: Genetic testing confirming the patient has a mutation of the DMD gene that is amenable to exon 45 skipping.

   d. Baseline renal function tests (i.e., glomerular filtration rate GFR)..
3. Patient has been stable on systemic corticosteroid regimen for at least 24 weeks, unless contraindicated or experienced significant adverse effects (must receive documentation)
4. Prescribed by or in consultation with a pediatric/adult neurologist or a physician who is an expert in the treatment of DMD, other neuromuscular disorders
5. Prescriber understands that continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials (PI)
6. Patient's kidney function will be evaluated before and during treatment as required by medication's label
7. Weight must be received for drugs that have weight-based dosing
8. Medication is prescribed in accordance with Food and Drug Administration (FDA) established indication and dosing regimens or in accordance with medically appropriate off-label indication and dosing according to American Hospital Formulary Service, Micromedex, Clinical Pharmacology, Lexi-Drugs, national guidelines, or other peer-reviewed evidence
9. Patient will not use golodirsen (Vyondys 53®) together with viltolarsen (Viltepso®)

Initial Approval: 6 months

Continuation of therapy:

1. Updated chart notes demonstrating positive clinical response to therapy (such as improvement and/or stabilization compared to baseline)
2. Prescribed by or in consultation with a pediatric/adult neurologist or a physician who is an expert in the treatment of DMD, other neuromuscular disorders
3. For dose increases, the member's weight must be received
4. Medication is prescribed in accordance with Food and Drug Administration (FDA) established indication and dosing regimens or in accordance with medically appropriate off-label indication and dosing according to American Hospital Formulary Service,
5. Micromedex, Clinical Pharmacology, Lexi-Drugs, national guidelines, or other peer-reviewed evidence
6. Patient will not use golodirsen (Vyondys 53®) together with viltolarsen (Viltepso®)

Renewal Approval: 6 months

References:

1. Exondys 51 [package insert]. Cambridge, MA: Sarepta Therapeutics, Inc.; September 2016.
2. Vyondys 53 [package insert]. Sarepta Therapeutics, Inc.; Cambridge, MA. March 2020.
3. Viltepso [package insert]. NS Pharma, Inc. Paramus, NJ 07652
4. Amondys 45 [package insert]. Sarepta Therapeutics, Inc; Cambridge MA. February 2021
5. Clinical Pharmacology [database online]. Tampa, FL: Gold Standard, Inc.: 2019. URL: http://www.clinicalpharmacology.com. Updated periodically
6. Mendell JR, et al; Eteplirsen Study Group. Eteplirsen for the treatment of Duchenne muscular dystrophy. Ann Neurol. 2013;74(5):637-647.

7. Lee JJA, Saito T et al. Direct Reprogramming of Human DMD Fibroblasts into Myotubes for In Vitro Evaluation of Antisense-Mediated Exon Skipping and Exons 45-55 Skipping Accomplished by Rescue of Dystrophin Expression. Methods Mol Biol. 2018; 1828: 141-150
8. Bushby K, Finkel R, Birnkrant DJ, Case LE, Clemens PR, Cripe L, et al. Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and pharmacological and psychosocial management. Lancet Neurol; 2010 Jan; 9(1):77 93.