Aetna Better Health of New Jersey

Addendum to Protocol for Duchenne Muscular Dystrophy Products

Approved July 2024

Addendum:
Addition of Agamree® (vamorolone) – FDA-approved October 26, 2023, and Emflaza protocol (previously DURB approved in July 2020)

Background:
Eteplirsen (Exondys 51),® Golodirsen (Vyondys 53®), Viltolarsen (Viltepso®), and Casimersen (Amondys 45®) are antisense oligonucleotides indicated for the treatment of Duchenne Muscular Dystrophy (DMD). Delandistrogene moxeparvovec-rokl (Elevidys®) is an adeno-associated virus vector-based gene therapy indicated for the treatment of ambulatory pediatric patients aged 4 through 5 years with DMD. Vamorolone (Agamree®) and Deflazacort (Emflaza) are corticosteroids indicated for the treatment of DMD.

Non-Preferred Agents:
Agamree (vamorolone)
Amondys 45 (casimersen)
Exondys 51 (eteplirsen)
Elevidys (delandistrogene moexeparvovec-rokl)
Vyondys 53 (golodirsen)
Viltepso (viltolarsen)

Criteria for Approval:

Antisense Oligonucleotides (Exondys 51, Vyondys 53, Viltepso, Amondys 45)

1. Patient must have a confirmed 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.

3. Patient is of the appropriate age based on FDA labeling or pharmaceutical compendia

4. Baseline renal function tests (i.e., glomerular filtration rate or GFR) as required by the medication label

5. Patient has been stable on a systemic corticosteroid regimen for at least 12 weeks, unless contraindicated, or has experienced significant adverse effects (documentation required)

6. Prescribed by or in consultation with a pediatric or adult neurologist or another specialist who is

an expert in the treatment of DMD and other neuromuscular disorders

7. Prescriber understands that continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials

8. Patient's kidney function will be evaluated before and during treatment as required by the medication label

9. Weight will be monitored for drugs that have weight-based dosing

10. Patient will not use in combination with another antisense oligonucleotide.

11. Medication is prescribed in accordance with a 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

Oral Glucocorticoids (Agamree, Emflaza):

1. Patient must have a confirmed diagnosis of Duchenne Muscular Dystrophy (DMD)

2. Patient is of the appropriate age based on FDA labeling or pharmaceutical compendia

3. Patient has history of trial and failure, intolerance or contraindication to at least a 3-month trial of prednisone

4. Prescribed by or in consultation with a pediatric or adult neurologist or another specialist who is an expert in the treatment of DMD or other neuromuscular disorders

5. Patient is monitored for the potential development of infection

6. Weight will be monitored for drugs that have weight-based dosing

7. Medication is prescribed in accordance with a 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

Gene Therapies (Elevidys):

1. Patient has a diagnosis of Duchenne Muscular Dystrophy (DMD) and genetic testing confirms that the patient has a mutation in the DMD gene, but not a deletion mutation in exon 8 and/or exon 9, since Elevidys is contraindicated for patients with these deletion mutations.
2. Baseline anti-AAVrh74 antibody titers <1:400 as determined by a total binding antibody ELISA

3. Baseline platelet counts, liver function tests, and troponin-I levels are obtained prior to initiating treatment

4. Elevidys will not be used in combination at the same time as the exon-skipping therapies (casimersen, eteplirsen, golodirsen, viltolarsen); however, it can be used after discontinuation of these treatment options.

5. Treatment is one time only

6. Patient is of the appropriate age based on FDA labeling or pharmaceutical compendia

7. Medication is prescribed in accordance with a 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

Continuation of therapy:

1. Updated chart notes demonstrate positive clinical response to therapy (such as improvement and/or stabilization compared to baseline)

2. Prescribed by or in consultation with a pediatric or adult neurologist or another specialist who is an expert in the treatment of DMD and other neuromuscular disorders

3. Patient is monitored for the potential development of infections

4. Medication is prescribed in accordance with a 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

5. Patient will not use golodirsen (Vyondys 53®) together with viltolarsen (Viltepso®)

Approval Duration and Quantity Restrictions:

- Elevidys - One month for a total of one dose
- All others - 6 months

Quantity Level Limit:

Agamree
- Available as 40mg/mL oral susp (100 mL per bottle) - 3 bottles (300 mL) per 30 days

Exondys 51
- Available as 100 mg/2 mL (50 mg/mL) single-dose vial - 120 vials (240 mL) per 28 days
- Available as 500 mg/10 mL (50 mg/mL) single-dose vial - 24 vials (240 mL) per 28 days

Vyondys 53
- Refer to product labeling at https://www.vyondys53.com/pi
- Available as 100 mg/2 mL single-dose vial

Viltepso
- Refer to product labeling at https://www.viltepso.com/prescribing-information
- Available as 250 mg/5 mL (50 mg/mL) single-dose vial

Amondys 45

- Refer to product labeling at https://www.amondys45.com/pi
- Available as 100 mg/2 mL single-dose vial

Elevidys

- Refer to product labeling at https://www.elevidys.com/downloads/elevidys-pi.pdf
- Member will not exceed a dose of 30 mg/kg once weekly

References:

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3. Viltepso [package insert]. NS Pharma, Inc. Paramus, NJ 07652
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