

Addendum to Protocol for Duchenne Muscular Dystrophy Products January 2026

Non-Preferred Agents

Exondys 51 (eteplirsen)

Vyondys 53 (golodirsen)

Viltepso (viltolarsen)

Amondys 45 (casimersen)

Elevidys (delandistrogene moxeparvovec-rokl)

Agamree (vamorolone)

deflazacort (Emflaza)

Duvyzat (givinostat)

Protocol applies to FDA approved biosimilars and related indications and dosages

Addendum:

Duvyzat, a new drug approved for Duchenne Muscular Dystrophy (DMD), has been added in this update. Monitoring parameters to assess kidney function and toxicity are updated for the antisense oligonucleotides. In addition, Elevidys is now only indicated for use in ambulatory patients with DMD.

Background:

Duchenne muscular dystrophy (DMD) is a progressive X-linked recessive neuromuscular disorder. The mutations in the DMD gene effects the dystrophin protein production. In patients with DMD, dystrophin is either absent or dysfunctional, which leads to progressive muscle degeneration. Dystrophin helps to keep the muscle cells intact and thereby preventing muscle breakdown. Exondys 51, Vyondys 53, Viltepso and Amondys 45 are antisense oligonucleotides that work by skipping the mutated section of the DMD gene. These drugs produce a truncated but functional dystrophin protein. Elevidys is the first gene therapy approved for DMD. It is an adeno-associated virus (AAV) based gene therapy indicated for the treatment of ambulatory patients with DMD. Agamree and Emflaza are corticosteroids indicated for the treatment of DMD. Duvyzat is a histone deacetylase (HDAC) enzyme inhibitor approved for use in patients 6 years of age and older. Inhibition of HDAC leads to a decrease in muscle inflammation and fibrosis.

Criteria for initial approval must meet the following:

1. Patient is of the FDA-labeled or compendial approved age
2. Patient does not have any contraindications to therapy
3. The medication requested is prescribed in accordance with a Food and Drug Administration (FDA) established indication and dosing regimens or in accordance with a medically-appropriate off-label indication and dosing according to American Hospital Formulary Service, Micromedex, Clinical Pharmacology, Wolters Kluwer Lexi-Drugs (Lexicomp), national guidelines, or other peer-reviewed evidence
4. The medication is prescribed by or in consultation with a neurologist or another specialist who is an expert in the treatment of DMD and other neuromuscular disorders
5. Patient has a confirmed diagnosis of Duchenne Muscular Dystrophy (DMD)
6. Patient was stable on a systemic corticosteroid regimen for at least 12 weeks, unless contraindicated, or had experienced significant adverse effects (documentation required)
7. For products with weight-based dosing, the patient's most current weight is provided

For Antisense Oligonucleotides (Exondys 51, Vyondys 53, Viltepso, Amondys 45) the patient meets all of the following criteria (1. through 4.):

1. Submission of documentation (e.g. medical records and labs) which includes 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
- 2. Prescriber plans to continue steroid treatment unless there are contraindications, intolerance or hypersensitivity
- 3. Prescriber acknowledges that continued approval for this indication may be contingent upon confirmatory trials
- 4. Patient is not using the requested medication concomitantly with another antisense oligonucleotide

Request for Vyondys 53, Viltepso, or Amondys 45, the patient will be monitored for kidney toxicity during treatment with the patient's urine sample measured prior to infusion or at least 48 hours after the most recent infusion

For Oral Glucocorticoids (Agamree, Emflaza) requests the patient meets all of the following:

- 1. Prescriber confirms the patient is up to date on immunizations
- 2. Prescriber attests any live or live-attenuated vaccines are administered at least 4 to 6 weeks prior to the first dose of requested medication

For Gene Therapy (Elevidys) requests the patient meets all of the following:

- 1. Genetic testing confirms that the patient has a mutation in the DMD gene
- 2. Documentation is received showing the patient does not have a deletion in exon 8 and/or exon 9
- 3. Patient has baseline anti-AAVrh74 antibody titers <1:400 as determined by a total binding antibody titers
- 4. Patient is ambulatory
- 5. The patient does not have any of the following:
 - a. Gamma-glutamyl transferase [GGT] >2 times the upper limit of normal
 - b. Total bilirubin > the upper limit of normal not due to Gilbert's syndrome
 - c. Active hepatic viral infection
 - d. Recent vaccination, within 4 weeks of Elevidys treatment
 - e. A current, active infection or infection within the previous 4 weeks
- 6. Post-Elevidys infusion the prescriber attests to monitoring all of the following:
 - a. Weekly platelets count for the first two weeks, continue monitoring if clinically necessary
 - b. Weekly liver function assessment (clinical exam, GGT, ALT, AST, aPTT, INR and total bilirubin) for the first 3 months and thereafter as clinically necessary
 - c. Weekly troponin-1 measurements for the first month, continue monitoring if clinically necessary
- 7. Elevidys is not used in combination with any exon-skipping therapies (casimersen, eteplirsen, golodirsen, viltolarsen); however, it can be used after discontinuation of these treatment options
- 8. Treatment is one-time only
- 9. The prescriber has informed the patient to stay in close proximity to an appropriate healthcare facility for at least 2 months after receiving Elevidys infusion

For Histone Deacetylase (HDAC) Inhibitor (Duvyzat) the patient meets all of the following:

- 1. Documentation is received confirming the patient has baseline platelet counts $\geq 150 \times 10^9/L$
- 2. Platelets are monitored every 2 weeks for the first 2 months, then monthly for the first 3 months and every 3 months thereafter post treatment

3. The prescriber is monitoring the following:
 - a. Patient's triglyceride levels at 1 month, 3 months, 6 months then every 6 months thereafter
 - b. ECGs if the patient has cardiac disease or is taking medications that causes QT prolongation
 - c. For the potential presence of moderate or severe diarrhea

Elevidys has a black box warning for acute serious liver injury, including life-threatening and fatal acute liver failure. See full prescribing information for complete boxed warning.

Continuation of therapy for all drugs except Elevidys (delandistrogene moxeparvovec-rokl):

1. Updated chart notes demonstrate positive clinical response to therapy (such as improvement and/or stabilization compared to baseline)
2. The medication is prescribed in accordance with a Food and Drug Administration (FDA) established indication and dosing regimens or in accordance with a medically-appropriate off-label indication and dosing according to American Hospital Formulary Service, Micromedex, Clinical Pharmacology, Wolters Kluwer (Lexicomp), national guidelines, or other peer-reviewed evidence
3. 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

Duvyzat

- Available as 8.86 mg/mL oral susp (140 mL per bottle): 3 bottles (420 mL) per 30 days

Deflazacort, Jaythari, and Kymbee

- Available as tablets 6 mg: 60 tablets per 30 days
- Available as tablets 18 mg: 30 tablets per 30 days
- Available as tablets 30 mg: 30 tablets per 30 days
- Available as tablets 36 mg: 30 tablets per 30 days
- Available as suspension 22.75 mg/mL: 52 mL per 30 days (1.8 mL/day)

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

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