

Duchenne Muscular Dystrophy

Goal(s):

- Encourage use of corticosteroids which have demonstrated long-term efficacy.
- Restrict use of targeted oligonucleotides for exon skipping to patients with Duchenne Muscular Dystrophy (DMD).
- Limit use of non-preferred corticosteroids to patients with contraindications or serious intolerance to preferred oral corticosteroids.
- Incorporate secondary review for drugs on the high-cost drug carve-out list.

Length of Authorization:

- 6-12 months (criteria-specific)

Requires PA:

- Targeted therapies for exon skipping or histone deacetylase (HDAC) inhibitors (see Table 1; pharmacy or provider administered claims)
- Corticosteroids that are FDA-approved for Duchenne muscular dystrophy (e.g., deflazacort, vamorolone, etc)

Covered Populations:

- Targeted therapies in Table 1: FFS and CCO enrolled populations beginning 1/1/26
- Corticosteroids: FFS populations only

Covered Alternatives:

- Current PMPDP preferred drug list per OAR 410-121-0030 at www.orpdl.org
- Searchable site for Oregon FFS Drug Class listed at www.orpdl.org/drugs/

Table 1. FDA Approved Indications for targeted therapies

Drug	Indication	Examples of amenable mutations (list is not all inclusive)	Recommended safety monitoring
casimersen (Amondys 45 [®])	Duchenne muscular dystrophy with mutations amenable to exon 45 skipping	Deletion of exons 44, 46, 46 to 47, 46 to 48, 46 to 49, 46 to 51, 46 to 53, 46 to 55, or 46 to 57	Renal function (e.g., serum cystatin C, urine dipstick, and urine protein-to-creatinine) within the past 3 months
eteplirsen (Exondys 51 [®])	Duchenne muscular dystrophy with mutations amenable to exon 51 skipping	Deletion of exons 43 to 50; 45 to 50; 47 to 50; 48 to 50; 49 to 50; 50; or 52	None
golodirsen (Vyondys 53 [®])	Duchenne muscular dystrophy with mutations amenable to exon 53 skipping	Deletion of exons 42 to 52; 45 to 52; 47 to 52; 48 to 52; 49 to 52; 50 to 52; 52; or 54 to 58	Renal function (e.g., serum cystatin C, urine dipstick, and urine protein-to-creatinine) within the past 3 months
viltolarsen (Viltepso [®])	Duchenne muscular dystrophy with mutations amenable to exon 53 skipping	Deletion of exons 42 to 52; 45 to 52; 47 to 52; 48 to 52; 49 to 52; 50 to 52; 52; or 54 to 58	
givinostat (Duvyzat [®])	Genetically confirmed Duchenne muscular dystrophy	No specific restrictions for type of mutation	Fasting triglycerides <300 mg/dL, platelet count > 150 x10 ⁹ cells/L for all patients,

			and ECG in people with heart disease or cardiac risk factors within the past 3 months
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Table 2. Minimum recommended givinostat doses

Weight	Minimum recommended dose
10 to <20 kg	13.3 mg twice daily
20 to <40 kg	17.7 mg twice daily
40 to <60 kg	26.6 mg twice daily
≥ 60 kg	35.4 mg twice daily

Approval Criteria		
1. What diagnosis is being treated?	Record ICD10 code.	
2. Is the request for treatment of Duchenne Muscular Dystrophy?	Yes: Go to #3	No: Pass to RPh. Deny; medical appropriateness. Note: Therapies are not indicated for other forms of muscular dystrophy or other diagnoses.
3. Is the request for a corticosteroid?	Yes: Go to #4	No: Go to #7
4. Is the patient ≥ 2 years of age?	Yes: Go to #5	No: Pass to RPh. Deny; medical appropriateness.
5. Has the patient received, or have contraindications to, all routine immunizations recommended for their age? Note: Routine vaccinations for patients at least 2 years of age typically include hepatitis B, hepatitis A, diphtheria, tetanus, pertussis, pneumococcal conjugate, inactivated poliovirus, influenza, and at least one dose of measles, mumps, rubella, and varicella.	Yes: Go to #6 Document physician attestation of immunization history.	No: Pass to RPh. Deny; medical appropriateness.
6. Does the patient have a documented contraindication or intolerance to a preferred corticosteroid, such as oral prednisone, that is not expected to crossover to the requested therapy? Note: deflazacort may be an option for patients with clinically significant weight gain associated with prednisone use.	Yes: Approve for up to 12 months. Document contraindication or intolerance reaction.	No: Pass to RPh. Deny; medical appropriateness. Recommend trial of prednisone.

Approval Criteria

7. Is the request for givinostat?	Yes: Go to #8	No: Go to #9
8. Is the requested dose at or above the minimum recommended FDA dose (Table 2)? Note: Discontinuation of givinostat is recommended if adverse events persist despite dose reduction. There is no evidence evaluating efficacy of lower doses.	Yes: Go to #9	No: Pass to RPh, Deny; medical appropriateness.
9. Is the request for continuation of treatment previously approved by FFS?	Yes: Go to Renewal Criteria	No: Go to #10
10. Is the request for an FDA-approved indication (Table 1)?	Yes: Go to #11 Document genetic testing.	No: Pass to RPh, Deny; medical appropriateness.
11. Is the request for combination treatment with 2 or more targeted therapies? There is no data evaluating combined use of targeted treatments for Duchenne Muscular Dystrophy.	Yes: Pass to RPh. Deny; medical appropriateness.	No: Go to #12
12. Has baseline testing been completed as recommended in the FDA label (Table 1)?	Yes: Go to #13	No: Pass to RPh. Deny; medical appropriateness.
13. Has the patient been on a stable dose of corticosteroid for at least 6 months or have documented contraindication to steroids?	Yes: Go to #14	No: Pass to RPh. Deny; medical appropriateness.
14. Has baseline functional assessment been evaluated using a validated tool (e.g., the 6-minute walk test, North Star Ambulatory Assessment, etc)?	Yes: Pass to RPh. Pend; Refer to DMAP for secondary review. Duration: Up to 6 months. Document baseline functional assessment.	No: Pass to RPh. Deny; medical appropriateness.

Renewal Criteria		
<p>1. Has the provider performed safety monitoring as recommended in the FDA label (Table 1)?</p> <p>Recommended monitoring includes urine dipstick monthly, serum cystatin C every 3 months, and protein-to-creatinine ratio every 3 months.</p>	<p>Yes: Go to #2</p>	<p>No: Pass to RPh, Deny; medical appropriateness.</p>
<p>2. Has the patient's baseline functional status been maintained at or above baseline level or not declined more than expected given the natural disease progression?</p>	<p>Yes: Go to #3</p> <p>Document functional status and provider attestation.</p>	<p>No: Pass to RPh, Deny; medical appropriateness.</p>
<p>3. Is there documentation based on chart notes of any serious adverse events related to treatment (e.g., acute kidney injury, infections, low platelets, high triglycerides, etc.)?</p>	<p>Yes: Go to #4</p>	<p>No: Approve for up to 6 months</p>
<p>4. Has the adverse event been reported to the FDA Adverse Event Reporting System (FAERS)?</p>	<p>Yes: Pass to RPh. Pend; Refer to DMAP for secondary review.</p> <p>Duration: Approvals cover up to 6 months</p> <p>Document provider attestation</p>	<p>No: Pass to RPh, Deny; medical appropriateness.</p>

P&T/DUR Review:

10/24, 2/24; 8/21 (SS); 2/21; 6/20; 09/19; 11/17; 07/17

Implementation:

12/1/2024; 4/1/24; 9/1/21; 3/1/21; 7/1/20; 11/1/19; 1/1/18; 9/1/17