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Drug Class Update: Duchenne Muscular Dystrophy

Date of Review: October 2026

Date of Last Review: October 2024
Dates of Literature Search: 07/01/2024 – 08/04/2026

Current Status of PDL Class:
See **Appendix 1**.

Purpose for Class Update:
Evaluate new evidence published since the last class review and to evaluate evidence pertaining to combination treatment with targeted therapies.

Plain Language Summary:
- People who have Duchenne muscular dystrophy (DMD) slowly lose muscle strength and ability to walk over time.
- The American Academy of Neurology recommends steroids, such as prednisone, for people with DMD because studies show that they extend the time people are able to walk and delay the need for a wheelchair. Studies do not show that one steroid improves muscle function better than another.
- In controlled studies, other medicines that the Food and Drug Administration (FDA) has approved for DMD have not shown that they improve symptoms or change the course of the disease.
- In 2025, the FDA issued new safety alerts related to a gene therapy for people with DMD. The name of this medicine is delandistrogene moxeparvovec. This medicine is no longer recommended for people with DMD who are not walking because two people who took the medicine died from liver failure. The FDA recommends weekly blood tests after this medicine is given in all patients to watch for early signs of liver problems.
- The Oregon Health Plan Open Card will currently pay for prednisone. Before Oregon FFS Medicaid will pay for other medicines in people with DMD, the provider must send in additional information to the Oregon Health Authority. This process is called prior authorization (PA).
- We recommend updating this policy to include new safety information for gene therapy. We also recommend that OHA not cover targeted medicines for DMD (other than steroids) after administration of gene therapy.

Research Questions:
1. What is the comparative efficacy or effectiveness of therapies for Duchenne muscular dystrophy (DMD) based on symptom improvement, muscle or pulmonary function, quality of life, or disease progression?
2. What is the comparative safety of therapies for people with DMD?
3. Are there any subgroups (based on age, gender, ethnicity, adjunct treatments, comorbidities, disease duration, or severity) that would benefit or be harmed from drugs for DMD?

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Conclusions:

- Since the last review of drugs for DMD, the FDA has strengthened warnings for acute liver failure associated with delandistrogene moxeparvovec following the deaths of two non-ambulatory patients who experienced acute liver failure following treatment.[1] The FDA is no longer recommending use of delandistrogene moxeparvovec in patients who are non-ambulatory.[1]
- There was no literature identified to evaluate efficacy and safety of combination treatment with gene therapy and other targeted treatments (e.g., histone deacetylase [HDAC] inhibitors or exon skipping therapy).

Recommendations:

- Update prior authorization criteria to align with updated indications and safety recommendations for delandistrogene moxeparvovec, and to prohibit use of targeted treatments after administration of gene therapy.

Summary of Prior Reviews and Current Policy

Therapies approved by the FDA for treatment of DMD were last reviewed by the Pharmacy and Therapeutics (P&T) Committee in October 2024.

- Corticosteroids are recommended as a first-line treatment for patients with DMD. Evidence from direct comparative randomized controlled trials (RCTs) shows no difference in efficacy between corticosteroids.[2,3] A RCT evaluating daily deflazacort or prednisone in people with DMD demonstrated comparable muscle and pulmonary function after 3 years.[2] Comparative efficacy data for vamorolone and prednisone is limited to 6 months.[3] Daily corticosteroid regimens (deflazacort or prednisone) were better at preserving muscle function than intermittent prednisone use.[2]
- Prior reviews have identified insufficient evidence to evaluate differences in safety between corticosteroids for DMD.[4,5] Deflazacort may be associated with less weight gain but more vision problems than prednisone.[2] There is insufficient evidence to evaluate whether vamorolone and prednisone have different effects on risk of fracture, growth, or development in people with DMD.[6]
- Exon-skipping treatments were approved by the FDA based on changes in dystrophin protein from baseline, and confirmatory studies have not been completed. Current evidence demonstrates no difference in motor function outcomes for exon-skipping therapies (e.g., casimersen, eteplirsen, golodirsen, viltolarsen) compared to placebo. Evidence is significantly limited by high risk of bias and small sample sizes.
- Two RCTs in patients treated with delandistrogene moxeparvovec have shown no difference in motor function compared to placebo after 48 and 52 weeks (low quality evidence).[7,8] In the initial placebo-controlled RCT used for accelerated approval, there was no statistical difference compared to placebo in the North Star Ambulatory Assessment (NSAA) score at 48 weeks (change from baseline of 1.7 vs. 0.9 points; least square mean difference [LSMD] 0.8; 95% confidence interval [CI] -1.03 to 2.67; p=0.37).[7] Secondary timed motor function tests were also no different between groups.[7] FDA approval was based on a post-hoc, subgroup analysis in people 4-5 years of age. The second confirmatory phase 3 RCT showed no statistically significant difference in the NSAA score compared to placebo (mean difference [MD] 0.65 points; 95% CI not reported, p=0.2441).[8] Results were not reported for all secondary motor outcomes, and when reported, had mean differences that were generally small.
- There is insufficient evidence that givinostat, a HDAC inhibitor, improves motor function outcomes in patients with DMD. When a mixed model for repeated measures was used to account for missing data, there was no difference between givinostat and placebo in the time to climb 4 stairs (MD -1.64 seconds, standard error [SE] 0.94, p=0.083).[9] Secondary motor outcomes including NSAA scores did not achieve statistical significance based on the pre-specified testing plan.[9,10] Common adverse events associated with givinostat include gastrointestinal disturbances (e.g., diarrhea, nausea, vomiting, abdominal pain, and decreased appetite), thrombocytopenia, increased triglycerides, pyrexia, rash, arthralgia, and fatigue.[11] Side effects were generally dose-related, and 42% of people had dose modifications due to adverse events compared to 8% of people treated with placebo.[9]

- PA is currently required for deflazacort, vamorolone, and all target therapies for DMD to ensure medically appropriate use (see **Appendix 4**). Prednisone is available without PA. Targeted therapies are carved-out of Coordinated Care Organizations and paid for by Open Card (or fee-for service).

Background:
Duchenne muscular dystrophy is a rare X-linked genetic disorder caused by the absence of a functional dystrophin protein. Duchenne muscular dystrophy primarily affects males and is the most common type of muscular dystrophy with an estimated worldwide prevalence of 1.7 to 4.2 in 100,000 patients.[12] Patients with DMD experience progressive muscle deterioration leading to loss of ambulation and decreased muscle strength. Disease progression varies considerably based on individual factors, and patients with Becker muscular dystrophy generally have less severe symptoms than people with DMD. Long-term complications for people with DMD include respiratory failure, dilated cardiomyopathy, arrhythmias, and increased risk for thrombotic events. In many patients, these complications can lead to wheelchair dependence by age 12 and death at an early age.[12] In a recent systematic review assessing median survival of patients with DMD, improved trends in survival have been documented over time.[13] Improved survival has been attributed to improvements in supportive care, including use of ventilator support, leading to a decrease in respiratory-associated deaths in this population.[13] Age of death in patients in earlier decades (e.g., 1960s-1970s), was significantly earlier than age of death for patients who died in more recent decades.[13] The pooled median survival was 29.9 years (95% CI 26.5 to 30.8) in patients with ventilator support compared to 19 years (95% CI 18 to 20.9) in patients without ventilator support.[13]

There is currently no curative treatment for DMD, and therapy focuses on improving symptoms, enhancing quality of life, and decreasing disease progression. Non-pharmacological therapies are often essential in disease management, and include physical therapy and use of support devices such as braces and wheelchairs.[12] As the disease progresses, mechanical ventilation and spinal surgery may be used to improve pulmonary function and decrease pain from scoliosis and vertebral fractures.[12] Guidelines from the American Academy of Neurology recommend initiation of corticosteroids as first-line treatment for ambulatory children with a decline in motor function to delay loss of ambulation, preserve pulmonary function, and reduce risk of scoliosis.[12,14] Corticosteroids are often continued if patients become non-ambulatory, though the continued benefits are less clear with progressive disease.[12] Some of the most common steroid regimens include prednisone 0.75 mg/kg/day, deflazacort 0.9 mg/kg/day, or intermittent prednisone 0.75 mg/kg for 10 day on and 10 days off for people unable to tolerate daily dosing.[2] Available evidence from direct comparative RCTs does not show any difference in efficacy between different corticosteroids for DMD.[2,3] However, daily corticosteroids may preserve muscle function better than intermittently dosed corticosteroids.[2]

Other treatments approved by the FDA for DMD include exon-skipping therapies, a HDAC inhibitor, and gene therapy. Exon-skipping therapies have been approved based on changes in dystrophin protein. The theoretical goal of these therapies is to modify mRNA splicing and increase the amount of dystrophin protein in cells, thereby correcting the underlying disease process. Using this mechanism, a truncated dystrophin protein is formed. While preclinical animal studies indicate truncated dystrophin can be functional, the level of function associated with the truncated protein is unknown and may vary depending on the inherited mutation.[15] While exon-skipping therapies have shown a slight increase in dystrophin, the impact of these therapies on clinical outcomes had not been demonstrated in RCTs.[16,17] There is currently no consensus on the minimum change in dystrophin level that may result in a clinical improvement, and available thresholds cited in the literature are currently based on expert opinion. An FDA analysis evaluating the change in 6-minute walk test (6MWT) per year and dystrophin level changes associated with golodirsen failed to demonstrate a positive correlation, indicating that small increases in a truncated dystrophin protein may not be an adequate surrogate marker for functional improvement.[18] Confirmatory post-marketing, randomized trials have not been completed for any exon skipping therapies.

Givinostat is a HDAC inhibitor which affects protein expression and regulation of follistatin.[9] The exact mechanism in DMD is unknown, but inhibition of HDAC in DMD is thought to increase muscle generation and reduce the fraction of healthy muscle cells that are replaced with fatty tissue or fibrosis. Approval was based

primarily on a double-blind, placebo-controlled phase 3 study evaluating motor function outcomes over 18 months.[10,11] The FDA also considered supplementary data from an observational, open-label extension study which compared efficacy of givinostat to natural history data in matched patients.[9]

Delandistrogene moxeparvovec is an adeno-associated viral vector-based gene therapy. The viral capsid contains a gene for an engineered, shortened micro-dystrophin protein which is delivered to muscle cells but not incorporated into the patient's DNA.[19] Because the gene encoding wild-type dystrophin is the largest known human gene, it is too large to be delivered in a viral capsid. Instead, the viral capsid contains select genetic sequences which are designed to encode a micro-dystrophin protein. This micro-dystrophin was based on protein identified in a patient with a milder form of the disease called Becker muscular dystrophy.[19] Micro-dystrophin proteins are not normally expressed in any patients, and it is not known if expression correlates with improved symptoms or a reduction in disease progression.[19] There is increasing literature which shows that dystrophin may play an important scaffolding role to recruit additional proteins necessary for normal muscle function such as ion channels, kinases, and neuronal nitric oxide synthase.[19] However, due to size constraints of the viral vector, the dystrophin protein regions responsible to these functions were not included in the micro-dystrophin formed by delandistrogene moxeparvovec.[19] Because the form of micro-dystrophin gene included in delandistrogene moxeparvovec is based on a form of dystrophin present in people with milder symptoms, the goal of this gene therapy is to reduce symptom severity not cure the disease.

Clinically important outcomes in DMD include morbidity, mortality, disease progression, motor function, and improvements in motor, pulmonary, or cardiac symptoms. There are multiple methods used assess motor function and strength in patients with DMD including timed functional tests and scoring tools. Examples include the NSAA which is a 17-item scale designed for patients able to ambulate at least 10 meters (total score range 0 to 34).[20,21] Other standard timed functional tests include time to climb 4 stairs, time to walk 10 meters, time required to stand from a supine position, and 6MWT which evaluates distance traveled in 6 minutes.[22] The minimum clinically important difference in the 6MWT for patients with DMD is approximately 30 meters.[22] NSAA scores less than 16 are more often correlated with 6MWT of less than 300 meters and scores greater than 30 correlate moderately with 6MWT of more than 400 meters.[21] The NSAA is generally considered a more comprehensive measure of functional status compared to other functional outcomes. Like all motor function assessments, NSAA score is often very dependent on motivation and method of administration.[23]

Methods:
A Medline literature search for new systematic reviews and RCTs assessing clinically relevant outcomes to active controls, or placebo if needed, was conducted. The Medline search strategy used for this review is available in **Appendix 2**, which includes dates, search terms and limits used. The OHSU Drug Effectiveness Review Project, Agency for Healthcare Research and Quality (AHRQ), National Institute for Health and Clinical Excellence (NICE), Department of Veterans Affairs, the Oregon Mental Health Clinical Advisory Group (MHCAG), the Scottish Intercollegiate Guidelines Network (SIGN), and Canada's Drug Agency (CDA-AMA) resources were manually searched for high quality and relevant systematic reviews. When necessary, systematic reviews are critically appraised for quality using the AMSTAR tool and clinical practice guidelines using the AGREE tool. The FDA website was searched for new drug approvals, indications, and pertinent safety alerts.

The primary focus of the evidence is on high quality systematic reviews and evidence-based guidelines. Randomized controlled trials will be emphasized if evidence is lacking or insufficient from those preferred sources.

New Systematic Reviews:
No new high quality systematic reviews were identified.

After review,4 systematic reviews were excluded due to poor quality (e.g., indirect network-meta analyses or failure to meet AMSTAR criteria), wrong study design of included trials (e.g., observational), comparator (e.g., no control or placebo-controlled), or outcome studied (e.g., non-clinical).[24-27]

New Guidelines:
High Quality Guidelines:
In 2026, NICE evaluated evidence for efficacy and safety of givinostat for treatment of DMD. Givinostat is recommended as a treatment option in people with DMD who are at least 6 years of age.[28]

In 2025, NICE evaluated evidence of efficacy and safety of vamorolone for people with DMD and recommended vamorolone as a treatment option in people at least 4 years of age.[29]

After review, one additional guideline was excluded due to poor quality.[30]

New Formulations or Indications:
In 2025, the FDA revised labeling for delandistrogene moxeparvovec to no longer include non-ambulatory patients with DMD following 2 cases of fatal liver injury in non-ambulatory patients.[31] Both patients presented with elevated transaminases and were hospitalized within 2 months after treatment.[31] Delandistrogene moxeparvovec was previously approved for non-ambulatory patients under the accelerated approval pathway based on expression of micro-dystrophin. The FDA also updated safety warnings and revised labeling to include these additional limitations: do not administer delandistrogene moxeparvovec in patients with pre-existing liver impairment, in patients with recent vaccinations (within 4 weeks of treatment) due to immunogenicity and potential safety concerns, or in patients with active or recent infections (within 4 weeks) due to safety concerns.[31]

New FDA Safety Alerts:

Table 1. Description of new FDA Safety Alerts

Generic Name	Brand Name	Month/Year of Change	Location of Change (Boxed Warning, Warnings, CI)	Addition or Change and Mitigation Principles (if applicable)
Delandistrogene moxeparvovec-rokl[31]	Elevidys	11/2025	FDA Safety Communication, Box Warning	Acute Serious Liver Injury and Liver Failure following treatment with delandistrogene moxeparvovec. In 2025, the FDA issued 2 safety communications and updated the box warning for acute serious liver injury and acute liver failure associated with delandistrogene moxeparvovec following 2 fatalities in non-ambulatory patients with DMD. Manufacturer labeling recommends monitoring liver function tests weekly for the at least the first 3 months after administration with continued monitoring until results are unremarkable. Corticosteroid dose adjustments and referral to a

				gastroenterologist or hepatologist are recommended if liver injury or abnormalities occur after administration. Administration in patients with active hepatic viral infection or pre-existing liver impairment is not recommended. Pre-existing liver impairment was defined as gamma-glutamyl transferase (GGT) more than two times the upper limit of normal or total bilirubin over the upper limit of normal. Additional liver function monitoring should aspartate aminotransferase (AST), alanine aminotransferase (ALT), albumin, activated partial thromboplastin time (aPTT), and international normalized ratio (INR). Administration in patients who are non-ambulatory, who have received recent vaccinations, or patients with recent infection is not recommended.

Randomized Controlled Trials:

A total of 33 citations were manually reviewed from the initial literature search. After further review, all citations were excluded because of wrong study design (e.g., observational), comparator (e.g., no control or placebo-controlled), or outcome studied (e.g., non-clinical).

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Appendix 1: Current Preferred Drug List

Generic	Brand	Form	PDL	Carveout
deflazacort	DEFLAZACORT	TABLET	Y	
deflazacort	EMFLAZA	TABLET	Y	
deflazacort	JAYTHARI	TABLET	Y	
deflazacort	KYMBEE	TABLET	Y	
casimersen	AMONDYS-45	VIAL	N	Y
deflazacort	DEFLAZACORT	ORAL SUSP	N	
deflazacort	EMFLAZA	ORAL SUSP	N	
deflazacort	JAYTHARI	ORAL SUSP	N	
deflazacort	KYMBEE	ORAL SUSP	N	
deflazacort	PYQUVI	ORAL SUSP	N	
delandistrogene moxeparvc-rokl	ELEVIDYS	KIT	N	Y
delandistrogene moxeparvc-rokl	ELEVIDYS	VIAL	N	Y
eteplirsen	EXONDYS-51	VIAL	N	Y
givinostat hydrochloride	DUVYZAT	ORAL SUSP	N	Y
golodirsen	VYONDYS-53	VIAL	N	Y
vamorolone	AGAMREE	ORAL SUSP	N	
viltolarsen	VILTEPSO	VIAL	N	Y

Appendix 2: Medline Search Strategy

Ovid MEDLINE(R) ALL 1946 to August 06, 2026

1	eteplirsen.mp.	215
2	golodirsen.mp.	79
3	exp Glucocorticoids/	218403
4	deflazacort.mp.	714
5	viltolarsen.mp.	69
6	vamorolone.mp.	78
7	casimersen.mp.	44
8	delandistrogene moxeparvovec.mp.	56
9	givinostat.mp.	191
10	delandistrogene moxeparvovec-rokl.mp.	6
11	exp Oligonucleotides, Antisense/	17746
12	duchenne muscular dystrophy.mp. or Muscular Dystrophy, Duchenne/	14957
13	1 or 2 or 3 or 4 or 5 or 6 or 7 or 8 or 9 or 10 or 11	236868
14	12 and 13	1245
15	limit 14 to (english language and humans and yr="2024 -Current")	170
16	limit 15 to (clinical trial, all or clinical trial, phase iii or clinical trial, phase iv or comparative study or controlled clinical trial or guideline or meta analysis or practice guideline or randomized controlled trial or "systematic review")	33

Appendix 3: Key Inclusion Criteria

Population	Patients with Duchenne Muscular Dystrophy
Intervention	Drugs in Appendix 1
Comparator	Drugs in Appendix 1
Outcomes	Motor function, quality of life, mortality, disease progression
Setting	Outpatient

Appendix 4: Prior Authorization Criteria

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	For all patients: Fasting triglycerides <300 mg/dL every 6 months, platelet count > 150 x10^9 cells/L ~~for all patients~~every 3 months, ~~and~~ For people with heart disease or cardiac risk factors: QTc evaluation (~~ECG~~electrocardiogram) at baseline and after initiating treatment ~~in people with heart disease or cardiac risk factors within the past 3 months~~

*For mutations not listed here, providers must submit documentation to show that the mutation is amenable to exon skipping.

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 an FDA-approved indication (Table 1)?	**Yes:** Go to #8 Document genetic testing.	**No:** Pass to RPh, Deny; medical appropriateness.
8. Is the request for continuation of a targeted treatment (Table 1) after administration of a gene therapy? Note: There is no data on efficacy or safety of targeted therapies after administration of gene therapy. Exon skipping therapies affect mRNA splicing and their impact on transcription of the micro-dystrophin gene delivered by the gene therapy is unknown.	**Yes:** Pass to RPh, Deny; medical appropriateness.	**No:** Go to #9
~~7.~~9. Is the request for givinostat?	**Yes:** Go to #~~8~~10	**No:** Go to #~~9~~11
~~8.~~10. 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~~ 11	**No:** Pass to RPh, Deny; medical appropriateness.
~~9.~~11. Is the request for continuation of treatment previously approved by FFS?	**Yes:** Go to **Renewal Criteria**	**No:** Go to #120~~0~~
~~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.~~12. 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 #132~~2~~

Approval Criteria		
~~12.~~13. Has baseline testing been completed as recommended in the FDA label (Table 1)?	**Yes**: Go to #1~~43~~	**No:** Pass to RPh. Deny; medical appropriateness.
~~13.~~14. Has the patient been on a stable dose of corticosteroid for at least 6 months or have documented contraindication to steroids?	**Yes:** Go to #1~~54~~	**No:** Pass to RPh. Deny; medical appropriateness.
~~14.~~15. 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		
1. Has the provider performed safety monitoring as recommended in the FDA label (Table 1)? ~~Recommended monitoring includes urine dipstick monthly, serum cystatin C every 3 months, and protein-to-creatine ratio every 3 months.~~ Note: givinostat dose adjustment is recommended for platelets <150x10^9/L; triglycerides >300 mg/dL; and QTc >500 or change from baseline >60 ms.	**Yes:** Go to #2	**No:** Pass to RPh, Deny; medical appropriateness.
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?	**Yes:** Go to #3 Document functional status and provider attestation.	**No:** Pass to RPh, Deny; medical appropriateness.

Renewal Criteria		
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.)?	**Yes:** Go to #4	**No:** Pass to RPh. Pend; Refer to DMAP for secondary review. Duration: Approvals cover up to 126 ~~6~~ months
4. Has the adverse event been reported to the FDA Adverse Event Reporting System (FAERS)?	**Yes:** Pass to RPh. Pend; Refer to DMAP for secondary review. Duration: Approvals cover up to ~~6~~ 12 months Document provider attestation	**No:** Pass to RPh, Deny; medical appropriateness.

P&T/DUR Review: *10/26 (SS); 10/24, 2/24; 8/21; 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*

Delandistrogene moxeparvovec

Goal(s):
- Restrict use of this gene therapy to patients with the FDA-labeled indication.
- Incorporate 2-step review process for drugs on the high-cost drug carve-out list.

Length of Authorization:
- 1 lifetime dose

Requires PA:
- Delandistrogene moxeparvovec (pharmacy and provider administered claims)

Covered Populations:
- FFS and CCO enrolled populations beginning 1/1/26

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/

Approval Criteria		
1. What diagnosis is being treated?	Record ICD10 code.	
2. Is the request for treatment of genetically-confirmed Duchenne Muscular Dystrophy?	**Yes:** Go to #3 Results of genetic testing are required for approval.	**No:** Pass to RPh. Deny; medical appropriateness. Note: Therapies are not indicated for other forms of muscular dystrophy or other diagnoses.
3. Is the patient currently ambulatory (e.g., able to complete a 6 minute walk test in the past 3 months)?	**Yes:** Go to #4	**No:** Pass to RPh. Deny; medical appropriateness.
~~3.~~4. Is the medication prescribed by a neuromuscular specialist?	**Yes:** Go to #~~4~~5	**No:** Pass to RPh. Deny; medical appropriateness.
~~4.~~5. Is the request for an FDA approved age (i.e., 4 years or older)?	**Yes:** Go to #~~5~~6	**No:** Pass to RPh. Deny; medical appropriateness.
~~5.~~6. Does the patient have deletions of exon 8 or 9?	**Yes:** Pass to RPh. Deny; medical appropriateness.	**No:** Go to #~~6~~7
~~6.~~7. For patients with deletions of exons 1 to 17 or exons 59 to 71, is there documentation that the provider and patient have discussed potential risks of treatment? Note: these populations were excluded from clinical studies and may have increased risk for severe immune-mediated myositis reactions.	**Yes:** Go to #~~7~~8	**No:** Pass to RPh. Deny; medical appropriateness.

Approval Criteria		
8. Is the intent to administer other targeted therapy for Duchenne muscular dystrophy after administration of the gene therapy? Note: There is no data on the efficacy or safety of combination treatment with gene therapy with givinostat, or exon-skipping therapies. Exon skipping therapies affect mRNA splicing and their impact on transcription of the micro-dystrophin gene delivered by gene therapy is unknown.	**Yes:** Pass to RPh. Deny; medical appropriateness.	**No:** Go to #9
~~7.~~9. Has baseline testing been completed within the past 3 months and is within normal limits? Recommended baseline testing includes testing for viral hepatitis (or documentation of immunization), anti-AAVrh74 antibodies (by ELISA), troponin-I, platelets, and liver function tests (e.g., AST, ALT, GGT, albumin, aPTT, INR, total bilirubin). Treatment is not recommended if GGT >2x the upper limit of normal, if total bilirubin is greater than the upper limit of normal, or if there is active hepatic viral infection.	**Yes:** Go to #~~8~~10 For any testing that is not within normal limits, refer to DMAP for secondary review. ~~Liver function tests should be <3x the upper limit of normal.~~	**No:** Pass to RPh. Deny; medical appropriateness.
~~8.~~10. Has the patient received, or have contraindications to, all routine immunizations recommended for their age? Note: Routine vaccinations for patients at least 4 years of age typically include hepatitis B, hepatitis A, diphtheria, tetanus, pertussis, pneumococcal conjugate, inactivated poliovirus, influenza, COVID-19, and at least 2 doses of measles, mumps, rubella, and varicella. Vaccines should not be administered within 4 weeks of gene therapy due to immunogenicity concerns.	**Yes:** Go to #~~9~~11 Document provider attestation of immunization history.	**No:** Pass to RPh. Deny; medical appropriateness.

Approval Criteria		
~~9.~~11. Is the patient able to tolerate an elevated dose of prednisone for at least 60 days and is there a documented plan to complete recommended weekly laboratory testing after administration~~necessary ongoing monitoring~~?	**Yes:** Go to #~~10~~12 Document provider attestation.	**No:** Pass to RPh. Deny; medical appropriateness.
~~10.~~12. Is there documentation that the provider and member have discussed potential risks of treatment? Note: Informed consent is recommended as this therapy has shown that it does not change global motor function in 2 clinical trials. It is associated with serious side effects including injury to the liver and heart and it may prevent use of any future adeno-based gene therapy.	**Yes**: Go to #131~~1~~	**No:** Pass to RPh. Deny; medical appropriateness.
~~11.~~13. Has the patient received a prior dose of an adeno-based gene therapy?	**Yes:** Pass to RPh. Deny; medical appropriateness.	**No:** Pass to RPh. Pend; Refer to DMAP for secondary review. Duration: Approvals cover one lifetime dose. Approval are valid for 12 months and will be extended if needed to cover treatment journey**.**

P&T/DUR Review: *10/26; 10/24, 2/24 (SS)*
Implementation: *12/1/2024; 4/1/24*