# Drugs for Duchenne Muscular Dystrophy

**Goal(s):**

- Encourage use of corticosteroids which have demonstrated long-term efficacy
- Restrict use of eteplirsen and deflazacort to patients with Duchenne Muscular Dystrophy and limit use of deflazacort to patients with contraindications or serious intolerance to other oral corticosteroids

**Length of Authorization:**

- 6 months

**Requires PA:**

- Eteplirsen (billed as a pharmacy or physician administered claim)
- Deflazacort

**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 drug being used to treat an OHP-funded condition AND is the requested treatment funded by the OHP for that condition?<br><br>Note: Treatments referenced on an unfunded line of the prioritized list (http://www.oregon.gov/oha/HPA/CSI-HERC/Pages/Prioritized-List.aspx) are not funded by the OHP. | **Yes:** Go to #3 | **No:** Pass to RPh. Deny; not funded by the OHP. |
| 3. Is the request for treatment of Duchenne Muscular Dystrophy? | **Yes:** Go to #4 | **No:** Pass to RPh. Deny; medical appropriateness.<br><br>Note: Eteplirsen and deflazacort are not indicated for other forms of muscular dystrophy or other diagnoses. |
| 4. Is the request for continuation of eteplirsen treatment? | **Yes:** Go to **Renewal Criteria** | **No:** Go to #5 |
| 5. Is the request for deflazacort? | **Yes:** Go to #6 | **No:** Go to #8 |

| Approval Criteria | | |
|---|---|---|
| 6. Is the patient ≥ 5 years of age? | **Yes:** Go to #7 | **No:** Pass to RPh. Deny; medical appropriateness. |
| 7. Does the patient have a documented contraindication or intolerance to oral prednisone that is not expected to crossover to deflazacort? | **Yes:** Approve for up to 12 months.<br><br>Document contraindication or intolerance reaction. | **No:** Pass to RPh. Deny; medical appropriateness.<br><br>Recommend trial of another oral corticosteroid. |
| 8. Does the patient have a diagnosis of Duchenne Muscular Dystrophy with one of the following genetic mutations amenable to exon 51 skipping:<br>• Deletion of exons 45 to 50<br>• Deletion of exons 48 to 50<br>• Deletion of exons 49 and 50<br>• Deletion of exon 50 OR<br>• Deletion of exon 52? | **Yes:** Go to #9<br><br>Document genetic testing. | **No:** Pass to RPh, Deny; medical appropriateness. |
| 9. Has the patient been on a stable dose of corticosteroid for at least 6 months? | **Yes:** Go to #10 | **No:** Pass to RPh. Deny; medical appropriateness. |
| 10. Has baseline functional assessment been evaluated using a validated tool such as the 6-minute walk test or North Star Ambulatory Assessment? | **Yes:** Document baseline functional assessment and approve for up to 6 months | **No:** Pass to RPh. Deny; medical appropriateness. |

| Renewal Criteria | | |
|---|---|---|
| 1. 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:** Approve for up to 6 months<br><br>Document functional status. | **No:** Pass to RPh, Deny; medical appropriateness. |

*P&T/DUR Review:* *11/17; 07/17 (SS)*
*Implementation:* *1/1/18; 9/1/17*