

## Montana Healthcare Programs Physician Administered Drug Coverage Criteria

### **EXONDYS 51® (eteplirsen)**

#### **I. Medication Description**

Exondys 51® is an antisense oligonucleotide indicated for:

Treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping.

#### **II. Position Statement**

Coverage is determined through a prior authorization process **that must include** supporting clinical documentation for each request.

#### **III. Initial Coverage Criteria**

Member must meet all the following criteria:

- Must have Duchenne muscular dystrophy (DMD) with a confirmed mutation of the DMD gene that is amenable to exon 51 skipping
  - The [www.duchenneconnect.org](http://www.duchenneconnect.org) website uses the following tool to find the genes amendable to Exon 51 skipping: <https://www.parentprojectmd.org/wp-content/exondeletiontool/>
  - Genetic mutation test results must be submitted with request.
- Must be prescribed by or in consult with a neurology specialist
- Must be on a stable dose of corticosteroids (prednisone, prednisolone, etc.) prior to starting Exondys 51®, unless corticosteroid use is contraindicated or was discontinued due to unfavorable side effects
- Corticosteroids (prednisone, prednisolone, etc.) must be used concurrently with Exondys 51®, unless corticosteroid use is contraindicated or was discontinued due to unfavorable side effects.
- If ambulatory, baseline functional level assessment required by one of the following:
  - Six-minute walk test (6MWT)
  - NorthStar Ambulatory Assessment
- If non-ambulatory, baseline functional level assessment required by one of the following:
  - Revised Upper Limb Module (RULM)
  - Performance Upper Limb (PUL)
- Exondys 51® is not used concomitantly with other exon skipping therapies for DMD.

#### **IV. Renewal Coverage Criteria**

Member must meet all the following criteria:

- Has been adherent to Exondys 51®
- Corticosteroids must be used concurrently, unless corticosteroid use is contraindicated or was discontinued due to unfavorable side effects.

- Functional level assessment must be completed every 6 months using the same rating scale used at baseline and submitted with renewal request.
- Member is receiving a benefit from Exondys 51® therapy, as demonstrated by one of the following:
  - Stabilization or improvement compared to baseline functional level assessment using the same rating scale submitted in initial approval.
  - Provider attests member requires continued use of medication, despite not meeting improved baseline functional level assessment criteria and the benefits of continued use of medication outweigh the risks.
- Annual specialist consult provided if prescriber not a specialist.

## **V. Quantity Limitations**

Max 30mg/kg IV once weekly

## **VI. Coverage Duration**

- Initial approval duration: 6 months
- Renewal approval duration: 6 months