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 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:
  o Stabilization or improvement compared to baseline functional level assessment using the same rating scale submitted in initial approval.
  o 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