

**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:

- Member 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 utilizes the following tool to find the genes amendable to exon 51 skipping: https://www.parentprojectmd.org/wp-content/exondeletiontool/](https://www.parentprojectmd.org/wp-content/exondeletiontool/)
  - Genetic mutation test results must be submitted with request.
- Exondys 51® must be prescribed by, or in consult with, a specialist familiar with DMD (usually a neurologist).
- Member 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.
- Member must be ambulatory (able to walk with assistance, and not wheelchair dependent).
- Exondys 51® is not used concomitantly with other exon skipping therapies for DMD.

**IV. Renewal Coverage Criteria**

Member must meet all the following criteria:

- Member has been adherent to Exondys 51®.
- Corticosteroids must be used concurrently, unless corticosteroid use is contraindicated, or was discontinued due to unfavorable side effects.
- Member must be ambulatory (able to walk with assistance, and not wheelchair dependent).

**V. Quantity Limitations**

Max 30mg/kg IV once weekly.

**VI. Coverage Duration**

Initial approval duration: 6 months

Renewal approval duration: 6 months