MONTANA HEALTHCARE PROGRAMS
Physician Administered Drug
Prior Authorization Criteria
Version 2, 02/14/2018

Exondys 51® (eteplirsen)

Covered Indications: Treatment of Duchenne Muscular Dystrophy (DMD) in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping (which affects about 13% of the population with DMD).

This indication was approved by the FDA based on an increase in dystrophin in skeletal muscle observed in some patients treated with Exondys 51®. A clinical benefit of Exondys 51® has not been established. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

Administration: 30 mg/kg administered once weekly as a 35 to 60 minute intravenous infusion

Initial Approval Criteria:

Limitations:

- Recipient must have Duchenne Muscular Dystrophy (DMD) with a confirmed mutation of the DMD gene that is amenable to exon 51 skipping. See Column 1 in attached Table 1 for DMD gene deletions that are potentially amenable to Exon 51 skipping. 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).
- Recipient 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.
- Recipient must be ambulatory (able to walk with assistance, and not wheelchair dependent).

Quantity Limit: 30 mg/kg administered once weekly

Duration of authorization: 6 months

Reauthorization Approval Criteria:

Limitations:

- Recipient must still be ambulatory (able to walk with assistance, and not wheelchair dependent).

Quantity Limit: 30 mg/kg administered once weekly

Duration of authorization: 6 months

Other Clinical Considerations (if applicable): Decisions will be rendered on a case-by-case basis based on individualized clinical information.

References:

1. EXONDYS 51® [package insert] Cambridge, MA; Sarepta Therapeutics, Inc.; 9/2016.

2. Darras, B. Treatment of Duchenne and Becker muscular dystrophy. Dashe, J, ed. UpToDate. Waltham, MA: UpToDate Inc. http://www.uptodate.com (accessed January 10, 2018).

3. https://www.duchenneconnect.org/component/content/article/114-website-content/decodeprogram/964-mutationspecific.html (accessed January 10, 2018)