Exondys 51 is an antisense oligonucleotide initially approved by the FDA in 2016. It is indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping. This indication is approved under accelerated approval based on an increase in dystrophin in skeletal muscle observed in some patients treated with Exondys 51. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

Exondys 51 (eteplirsen) will be considered for coverage when the following criteria are met:

**DUCHENNE MUSCULAR DYSTROPHY (DMD)**

For **initial** authorization:
1. Member has a diagnosis of DMD with confirmed mutation of DMD gene that is amenable to exon 51 skipping (genetic testing results required); AND
2. Medication is being prescribed by or in consultation with a DMD specialist (i.e., neurologist); AND
3. Member is currently stable on corticosteroid for at least 6 months prior to starting therapy, unless not tolerated or contraindicated; AND
4. Member’s body weight within the last 30 days has been submitted.
5. **Dosage allowed/Quantity limit:** 30 mg per kg of body weight once weekly.

*If all the above requirements are met, the medication will be approved for 6 months.*

For **reauthorization**:
1. Chart notes must show stability or slowed rate of decline of the member’s motor function compared to baseline.

*If all the above requirements are met, the medication will be approved for an additional 12 months.*

CareSource considers Exondys 51 (eteplirsen) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.
<table>
<thead>
<tr>
<th>Date</th>
<th>Action</th>
</tr>
</thead>
<tbody>
<tr>
<td>05/20/2019</td>
<td>Criteria on member’s ambulatory status and independent walking ability added to initial authorization and reauthorization parts of the policy.</td>
</tr>
<tr>
<td>06/23/2020</td>
<td>Length of corticosteroid trial specified to be at least 3 months.</td>
</tr>
<tr>
<td>01/14/2021</td>
<td>Added prescriber requirement. Simplified ambulatory requirement. Added requirement of stability or slowed rate of decline of motor function in reauth section.</td>
</tr>
<tr>
<td>04/06/2021</td>
<td>Increased duration of steroid trial to 6 months.</td>
</tr>
<tr>
<td>03/03/2023</td>
<td>Transferred to new template. Removed ambulatory requirement from reauth. Added weight requirement to ensure accurate dosing. Updated references. Removed ambulatory requirement from initial auth.</td>
</tr>
</tbody>
</table>

References:

2. Sarepta Therapeutics. An Open-Label, Multi-Center Study to Evaluate the Safety and Tolerability of Eteplirsen in Patients With Advanced Stage Duchenne Muscular Dystrophy. NLM Identifier: NCT02286947.

Effective date: 04/14/2023
Revised date: 03/03/2023