CareSource

PHARMACY POLICY STATEMENT
Marketplace

DRUG NAME	Exondys 51 (eteplirsen)
BILLING CODE	J1428 (1 unit = 10 mg)
BENEFIT TYPE	Medical
SITE OF SERVICE ALLOWED	Office/Outpatient/Home
COVERAGE REQUIREMENTS	Prior Authorization Required (Non-Preferred Product) QUANTITY LIMIT— see dosage allowed
LIST OF DIAGNOSES CONSIDERED **NOT** MEDICALLY NECESSARY	Click Here

Exondys 51 (eteplirsen) is a **non-preferred** product and will only be considered for coverage under the **medical** benefit when the following criteria are met:

Members must be clinically diagnosed with one of the following disease states and meet their individual criteria as stated.

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. Chart notes have been provided to show that the member is able to walk independently without assistive devices.
5. **Dosage allowed:** 30 mg per kg of body weight once weekly.

If member meets all the requirements listed above, 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; AND
2. Chart notes confirm that member remains able to walk independently without assistive devices.

If member meets all the reauthorization requirements above, the medication will be approved for an additional 6 months.

CareSource considers Exondys 51 (eteplirsen) not medically necessary for the treatment of the diseases that are not listed in this document.

DATE	ACTION/DESCRIPTION
11/29/2016	Last revision of the policy.
10/16/2017	Policy converted into new format. No changes in criteria.
05/20/2019	Criteria on member's ambulatory status and independent walking ability added to initial authorization and reauthorization parts of the policy.
06/23/2020	Length of corticosteroid trial specified to be at least 3 months.
01/14/2021	Added prescriber requirement. Simplified ambulatory requirement. Added requirement of stability or slowed rate of decline of motor function in reauth section.

04/06/2021	Increased duration of steroid trial to 6 months.

References:

1. Exondys 51 [Package Insert]. Cambridge, MA: Sarepta Therapeutics, Inc.; July 2020.
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.
3. Birnkrant DJ, Bushby K, Bann CM, et al. Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and neuromuscular, rehabilitation, endocrine, and gastrointestinal and nutritional management [published correction appears in Lancet Neurol. 2018 Apr 4;:]. *Lancet Neurol*. 2018;17(3):251-267.
4. Gloss D, Moxley RT 3rd, Ashwal S, Oskoui M. Practice guideline update summary: Corticosteroid treatment of Duchenne muscular dystrophy: Report of the Guideline Development Subcommittee of the American Academy of Neurology. *Neurology*. 2016;86(5):465-472.

Effective date: 10/1/2021
Revised date: 04/06/2021