HAP CareSource™

PHARMACY POLICY STATEMENT
HAP CareSource™ Marketplace

DRUG NAME	Exondys 51 (eteplirsen)
BILLING CODE	J1428
BENEFIT TYPE	Medical
SITE OF SERVICE ALLOWED	Home
STATUS	Prior Authorization Required

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.

HAP 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.

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.
03/02/2022	Transferred to new template. Removed ambulatory requirement. Added weight requirement to ensure accurate dosing. Updated references.
03/31/2023	Removed ambulatory requirement from initial criteria.

References:

1. Exondys 51 [Package Insert]. Cambridge, MA: Sarepta Therapeutics, Inc.; Jan 2022.
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: 01/01/2025
Revised date: 03/31/2023