PHARMACY POLICY STATEMENT
North Carolina 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 sk10.5 (m(s)p8.5 (m)-6 (enabl)2(ar)4.910.5)-2 nzathat iy appr2mmiaccelerated ap 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:

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.

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: 01/01/2023
Revised date: 03/02/2022