

Exondys-51™ (eteplirsen) **(Intravenous)**

Effective Date: 12/01/2019

**Review Date: 11/27/2019, 1/29/2020, 04/29/2021, 2/24/2022, 8/25/2022, 2/1/2023, 9/14/2023,
12/07/2023, 01/04/2024**

Scope: Medicaid, Commercial, Medicare-Medicaid Plan (MMP)

I. Length of Authorization

Coverage will be for 6 months and may be renewed.

II. Dosing Limits

A. Quantity Limit (max daily dose) [NDC Unit]:

- Exondys-51 100 mg/2ml single-dose vial: 4 vials per 7 days
- Exondys-51 500 mg/10ml single-dose vial: 7 vials per 7 days

B. Max Units (per dose and over time) [HCPCS Unit]:

- 350 billable units (3500mg) every 7 days

III. Summary of Evidence

Exondys 51 is an antisense oligonucleotide 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. Clinical trials have demonstrated an increase in dystrophin in skeletal muscle in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping. Adverse events commonly reporting in clinical trials include balance disorder and vomiting.

IV. Initial Approval Criteria ¹⁻¹⁰

Coverage is provided in the following conditions:

Universal Criteria

MMP members who have previously received this medication within the past 365 days are not subject to Step Therapy Requirements.

- Patient is not on concomitant therapy with other DMD-directed antisense oligonucleotides (e.g., golodirsen, casimersen, viltolarsen, etc.); **AND**
- Patient has never received and will not receive therapy with Elevidys (delandistrogene moxeparvovec-rokl) within 6 months of this request; **AND**

Duchenne Muscular Dystrophy (DMD) † Φ

- Patient must have a confirmed mutation of the *DMD* gene that is amenable to exon 51 skipping; **AND**
- Patient has been on a stable dose of corticosteroids, unless contraindicated or intolerance, for at least 6 months; **AND**
- Patient retains meaningful voluntary motor function (e.g., patient is able to speak, manipulate objects using upper extremities, ambulate, etc.); **AND**
- Patient is receiving physical and/or occupational therapy; **AND**
- Baseline documentation of one or more of the following:
 - Dystrophin level
 - Timed function tests (e.g., 6-minute walk test (6MWT) time to stand [TTSTAND], time to run/walk 10 meters [TTRW], time to climb 4 stairs [TTCLIMB], etc.)
 - Upper limb function (ULM) test
 - North Star Ambulatory Assessment (NSAA) score
 - Forced Vital Capacity (FVC) percent predicted

† FDA-labeled indication(s); ‡ Compendia recommended indication(s); Φ Orphan Drug

V. Renewal Criteria ¹

- Patient continues to meet universal and other indication-specific relevant criteria such as concomitant therapy requirements (not including prerequisite therapy), performance status, etc. identified in section III; **AND**
- Absence of unacceptable toxicity from the drug. Examples of unacceptable toxicity include: hypersensitivity reactions (e.g., bronchospasm, chest pain, cough, tachycardia, urticaria, etc.), etc.; **AND**

- Patient has responded to therapy compared to pretreatment baseline in one or more of the following (not all-inclusive):
 - Increase in dystrophin level
 - Improvement in quality of life
 - Stability, improvement, or slowed rate of decline in one of the following:
 - Timed function tests (e.g., 6-minute walk test [6MWT], time to stand [TTSTAND], time to run/walk 10 meters [TTRW], time to climb 4 stairs [TTCLIMB])
 - Upper limb function (ULM) test
 - North Star Ambulatory Assessment (NSAA) score
 - Forced Vital Capacity (FVC) percent predicted

VI. Dosage/Administration

Indication	Dose
Duchenne Muscular Dystrophy	30 mg/kg via intravenous infusion once weekly

VII. Billing Code/Availability Information

HCPCS Code:

- J1428 - Injection, eteplirsen, 10 mg; 1 billable unit = 10 mg

NDC:

- 100 mg/2 mL single-dose vial: 60923-0363-xx
- 500 mg/10 mL single-dose vial: 60923-0284-xx

VIII. References

1. Exondys 51 [package insert]. Cambridge, MA; Sarepta Therapeutics, Inc.; March 2022. Accessed November 2023.
2. Mendell JR, Rodino-Klapac LR, Sahenk Z, et al. Eteplirsen for the treatment of Duchenne muscular dystrophy. *Ann Neurol*. 2013 Nov;74(5):637-47. doi: 10.1002/ana.23982.

3. Mendell JR, Goemans N, Lowes LP, et al. Longitudinal effect of eteplirsen versus historical control on ambulation in Duchenne muscular dystrophy. *Ann Neurol*. 2016 Feb;79(2):257-71. doi: 10.1002/ana.24555.
4. Alfano LN, Charleston JS, Connolly AM, et al. Long-term treatment with eteplirsen in nonambulatory patients with Duchenne muscular dystrophy. *Medicine (Baltimore)*. 2019 Jun;98(26):e15858. doi: 10.1097/MD.00000000000015858.
5. Topaloglu H, Gloss D, Moxley RT 3rd, et al. Practice guideline update summary: Corticosteroid treatment of Duchenne muscular dystrophy: Report of the Guideline Development Subcommittee of the American Academy of Neurology. *Neurology*. 2016 Jul 12;87(2):238.
6. Bushby K, Finkel R, Birnkrant DJ, et al. Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and pharmacological and psychosocial management. *Lancet Neurol*; 2010 Jan; 9(1):77-93.
7. Bushby K, Finkel R, Birnkrant DJ, et al. Diagnosis and management of Duchenne muscular dystrophy, part 2: implementation of multidisciplinary care. *Lancet Neurol*; 2010 Jan; 9(2):177-189.
8. Kinane TB, Mayer OH, Duda PW, et al. Long-Term Pulmonary Function in Duchenne Muscular Dystrophy: Comparison of Eteplirsen-Treated Patients to Natural History. *Journal of Neuromuscular Diseases* 5 (2018) 47–58.
9. Institute for Clinical and Economic Review. Deflazacort, Eteplirsen, and Golodirsen for Duchenne Muscular Dystrophy: Effectiveness and Value. Final Evidence Report. August 15, 2019 https://icer-review.org/wp-content/uploads/2018/12/ICER_DMD-Final-Report_081519-1.pdf. Accessed December 2019.
10. Khan N, Eliopoulos H, et al on behalf of the Eteplirsen Investigators and the CINRG DNHS Investigators. Eteplirsen Treatment Attenuates Respiratory Decline in Ambulatory and Non-Ambulatory Patients with Duchenne Muscular Dystrophy. *J. Neuromuscular Dis*, vol. 6, no. 2, pp. 213-225, 2019.

Appendix 1 – Covered Diagnosis Codes

ICD-10	ICD-10 Description
G71.01	Duchenne or Becker muscular dystrophy

Appendix 2 – Centers for Medicare and Medicaid Services (CMS)

Medicare coverage for outpatient (Part B) drugs is outlined in the Medicare Benefit Policy Manual (Pub. 100-2), Chapter 15, §50 Drugs and Biologicals. In addition, National Coverage Determination (NCD), Local Coverage Articles (LCAs) and Local Coverage Determinations (LCDs) may exist and compliance with these policies is required where applicable. They can be found at: <http://www.cms.gov/medicare-coverage-database/search/advanced-search.aspx>. Additional indications may be covered at the discretion of the health plan.

Medicare Part B Covered Diagnosis Codes (applicable to existing NCD/LCA/LCD): N/A

Medicare Part B Administrative Contractor (MAC) Jurisdictions		
Jurisdiction	Applicable State/US Territory	Contractor
E (1)	CA, HI, NV, AS, GU, CNMI	Noridian Healthcare Solutions, LLC
F (2 & 3)	AK, WA, OR, ID, ND, SD, MT, WY, UT, AZ	Noridian Healthcare Solutions, LLC
5	KS, NE, IA, MO	Wisconsin Physicians Service Insurance Corp (WPS)
6	MN, WI, IL	National Government Services, Inc. (NGS)
H (4 & 7)	LA, AR, MS, TX, OK, CO, NM	Novitas Solutions, Inc.
8	MI, IN	Wisconsin Physicians Service Insurance Corp (WPS)
N (9)	FL, PR, VI	First Coast Service Options, Inc.
J (10)	TN, GA, AL	Palmetto GBA, LLC
M (11)	NC, SC, WV, VA (excluding below)	Palmetto GBA, LLC
L (12)	DE, MD, PA, NJ, DC (includes Arlington & Fairfax counties and the city of Alexandria in VA)	Novitas Solutions, Inc.
K (13 & 14)	NY, CT, MA, RI, VT, ME, NH	National Government Services, Inc. (NGS)
15	KY, OH	CGS Administrators, LLC

Policy Rationale: Exondys51 was reviewed by the Neighborhood Health Plan of Rhode Island Pharmacy & Therapeutics (P&T) Committee. Neighborhood adopted the following clinical coverage criteria to ensure that its members use Exondys51 according to Food and Drug Administration (FDA) approved labeling and/or relevant clinical literature. Neighborhood worked with network prescribers and pharmacists to draft these criteria. These criteria will help ensure its members are using this drug for a medically accepted indication, while minimizing the risk for adverse effects and ensuring more cost-effective options are used first, if applicable and appropriate. For INTEGRITY (Medicare-Medicaid Plan) members, these coverage criteria will only apply in the absence of National Coverage Determination (NCD) or Local Coverage Determination (LCD)

criteria. Neighborhood will give individual consideration to each request it reviews based on the information submitted by the prescriber and other information available to the plan.