

Exondys-51™ (eteplirsen) (Intravenous)

Document Number: MH-0284

Last Review Date: 08/03/2021

Date of Origin: 11/22/2016

Dates Reviewed: 11/2016, 10/2017, 08/2018, 10/2018, 08/2019, 02/2020, 08/2020, 08/2021

Customization Dates: 04/01/2022

Effective Dates: 04/01/2022

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 vial: 4 vials per 7 days
- Exondys-51 500 mg vial: 7 vials per 7 days

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

- 350 billable units every week

III. Initial Approval Criteria ¹⁻¹⁰

Coverage is provided in the following conditions:

Patient is required to meet Site of Service specialty infusion program requirements (refer to the [Medica Site of Service Policy](#)).

Submission of medical records (chart notes) related to the medical necessity criteria is **REQUIRED** on all requests for authorizations. Records will be reviewed at the time of submission. Please provide documentation related to diagnosis, step therapy, and clinical markers (i.e. genetic and mutational testing) supporting initiation when applicable. Medical records may be submitted via direct upload through the PA web portal or by fax.

Universal Criteria

- Patient is not on concomitant therapy with other DMD-directed antisense oligonucleotides (e.g., golodirsen, casimersen, viltolarsen, etc.); **AND**

Duchenne muscular dystrophy (DMD) † ☐

- Patient initiated on therapy prior to the age of 14 years old; AND
- Patient able to ambulate at least 180 meters while walking independently over 6 minutes within 4 weeks of initiation of therapy; AND

- 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 should be receiving physical and/or occupational therapy; AND
- Baseline documentation of one or more of the following:
 - Dystrophin level
 - 6-minute walk test (6MWT) or other timed function tests (e.g., time to stand [TTSTAND], time to run/walk 10 meters [TTRW], time to climb 4 stairs [TTCLIMB])
 - North Star Ambulatory Assessment (NSAA)
 - Forced Vital Capacity (FVC) percent predicted

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

IV. 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 the following: severe hypersensitivity reactions, 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
 - Stability, improvement, or slowed rate of decline in 6MWT or other timed function tests (e.g., time to stand [TTSTAND], time to run/walk 10 meters [TTRW], time to climb 4 stairs [TTCLIMB])
 - Stability, improvement, or slowed rate of decline in NSAA
 - Stability, improvement, or slowed rate of decline in FVC% predicted

V. Dosage/Administration

Indication	Dose
Duchenne muscular dystrophy	30 mg/kg via intravenous infusion once weekly

VI. Billing Code/Availability Information

HCP/PCS 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

VII. References

1. Exondys 51 [package insert]. Cambridge, MA; Sarepta Therapeutics, Inc.; July 2020. Accessed July 2021.
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