



Exondys-51™ (eteplirsen) (Intravenous)

Document Number: IC-0284

Last Review Date: 10/02/2018

Date of Origin: 11/22/2016

Dates Reviewed: 11/2016, 10/2017, 08/2018, 10/2018

I. Length of Authorization

Authorization is valid for 6 months and may be renewed.

II. Dosing Limits

A. Quantity Limit (max daily dose) [Pharmacy Benefit]:

- 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) [Medical Benefit]:

Duchenne muscular dystrophy

- 350 billable units every week

III. Initial Approval Criteria

Coverage is provided in the following conditions:

Duchenne muscular dystrophy (DMD) †

- 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 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 should be receiving physical therapy; **AND**
- Baseline documentation of one or more of the following:
 - Dystrophin level
 - 6-minute walk test (6MWT)

† FDA-labeled indication(s)

IV. Renewal Criteria

- Patient continues to meet the criteria in Section III; **AND**
- Absence of unacceptable toxicity from the drug. Examples of unacceptable toxicity include the following: severe hypersensitivity reactions, balance disorders, vomiting, 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
 - Improvement in quality of life

V. Dosage/Administration

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

Store refrigerated at 2°C to 8°C

VI. Billing Code/Availability Information

Jcode:

- 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.; February 2018. Accessed September 2018.
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. 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.

5. 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.
6. 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.

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) 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/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 Government Benefit Administrators, 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