Neighborhood Health Plan OF RHODE ISLAND™
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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, 08/28/2024
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., Vyondys 53 (golodirsen), Amondys 45 (casimersen), Viltepso (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 intolerant, 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 IV; **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 intravenously once weekly

VII. Billing Code/Availability Information

HCPCS Code:

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

NDC:

- Exondys 51 100 mg/2 mL single-dose vial: 60923-0363-xx
- Exondys 51 500 mg/10 mL single-dose vial: 60923-0284-xx

VIII. References

1. Exondys 51 [package insert]. Cambridge, MA; Sarepta Therapeutics, Inc.; January 2022. Accessed July 2024.
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11. 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. Lancet Neurol 2018; 17:251.
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of the American Academy of Neurology. Neurology. 2016 Feb 2;86(5):465-72. Doi: 10.1212/WNL.0000000000002337. Reaffirmed on January 22, 2022.

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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)

The preceding information is intended for non-Medicare coverage determinations. 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 Determinations (NCDs) and/or Local Coverage Determinations (LCDs) may exist and compliance with these policies is required where applicable. Local Coverage Articles (LCAs) may also exist for claims payment purposes or to clarify benefit eligibility under Part B for drugs which may be self-administered. The following link may be used to search for NCD, LCD, or LCA documents: https://www.cms.gov/medicare-coverage-database/search.aspx. Additional indications, including any preceding information, may be applied 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,	Noridian Healthcare Solutions, LLC
5	KS, NE, IA, MO	Wisconsin Physicians Service Insurance Corp (WPS)
6	MN, WI, IL	National Government Services, Inc. (NGS)

Medicare Part B Administrative Contractor (MAC) Jurisdictions		
Jurisdiction	**Applicable State/US Territory**	**Contractor**
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
M (11)	NC, SC, WV, VA (excluding below)	Palmetto GBA
L (12)	DE, MD, PA, NJ, DC (includes Arlington & Fairfax counties and the city of	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.