

# Exondys 51® (eteplirsen) (Intravenous)

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## I. Length of Authorization

- Initial: Prior authorization validity will be provided initially for 6 months (180 days).
- Renewal: Prior authorization validity may be renewed every 12 months (365 days) thereafter.

## II. Dosing Limits

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

- 1400 billable units every 28 days

## III. Initial Approval Criteria

Prior authorization validity is provided in the following conditions:

### Universal Criteria <sup>1</sup>

- Member is not on concomitant therapy with other DMD-directed antisense oligonucleotides; **AND**
- Member is not on concomitant therapy with delandistrogene moxeparvovec-rokl; **AND**

### Duchenne Muscular Dystrophy (DMD) † Φ <sup>1-15</sup>

- Member has a confirmed mutation of the *DMD* gene that is amenable to exon 51 skipping; **AND**
- Member has been on a stable dose of corticosteroids, unless there is a contraindication or intolerance, for at least 6 months; **AND**
- Member retains meaningful voluntary motor function (e.g., member is able to speak, manipulate objects using upper extremities, ambulate, etc.); **AND**
- Member 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 Approved Indication(s); ‡ Compendia Recommended Indication(s); Ⓞ Orphan Drug

#### IV. Renewal Criteria <sup>1-17</sup>

Prior authorization validity may be renewed based upon the following criteria:

- Member continues to meet the 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**
- Member 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], etc.)
    - Upper limb function (ULM) test
    - North Star Ambulatory Assessment (NSAA) score
    - Forced Vital Capacity (FVC) percent predicted

#### V. Dosage/Administration <sup>1</sup>

| Indication                  | Dose  |
|-----------------------------|---|
| Duchenne Muscular Dystrophy | Administer 30 mg/kg intravenously once weekly |

#### VI. Billing Code/Availability Information

HCPCS Code:

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

NDC(s):

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

#### VII. References

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13. Moxley RT 3<sup>rd</sup>, Ashwal S, Pandya S, et al. Practice parameter: corticosteroid treatment of Duchenne dystrophy: report of the Quality Standards Subcommittee of the American Academy of Neurology and the Practice Committee of the Child Neurology Society. *Neurology*. 2005;64:13–20.
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## Appendix A – Non-Quantitative Treatment Limitations (NQL) Factor Checklist

Non-quantitative treatment limitations (NQLs) refer to the methods, guidelines, standards of evidence, or other conditions that can restrict how long or to what extent benefits are provided under a health plan. These may include things like utilization review or prior authorization. The utilization management NQL applies comparably, and not more stringently, to mental health/substance use disorder (MH/SUD) Medical Benefit Prescription Drugs and medical/surgical (M/S) Medical Benefit Prescription Drugs. The table below lists the factors that were considered in designing and applying prior authorization to this drug/drug group, and a summary of the conclusions that Prime’s assessment led to for each.

| Factor                     | Conclusion            |
|----------------------------|-----------------------|
| Indication                 | Yes: Consider for PA  |
| Safety and efficacy        | No: PA not a priority |
| Potential for misuse/abuse | No: PA not a priority |
| Cost of drug               | Yes: Consider for PA  |

## 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

| <b>Medicare Part B Administrative Contractor (MAC) Jurisdictions</b> |   |   |
|--|---|---|
| <b>Jurisdiction</b>  | <b>Applicable State/US Territory</b>  | <b>Contractor</b>                                 |
| 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                                      |
| 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 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                           |