Neighborhood Health Plan OF RHODE ISLAND™
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Vyondys 53™ (golodirsen)
(Intravenous)

Effective Date: 03/06/2020
Review Date: 03/06/2020, 11/02/2020, 7/15/2021, 9/2/2021, 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

Authorization is valid for 6 months and may be renewed.

II. Dosing Limits

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

- Vyondys 53 100 mg/2ml single-dose vial: 35 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

Vyondys 53 (golodirsen) is indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the dystrophin gene that is amenable to exon 53 skipping. Clinical studies have shown to increase dystrophin production in muscle tissue, which is crucial for maintaining muscle integrity. This increase in dystrophin production is indicative of the drug's mechanism of action, exon skipping, which allows for the production of a truncated but partially functional dystrophin protein. Common adverse events including injection-site reactions, vomiting, and rash.

IV. Initial Approval Criteria [1-9]

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

Coverage is provided in the following conditions:

- Patient is not on concomitant therapy with other DMD-directed antisense oligonucleotides (e.g., Exondys 51 (eteplirsen), 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**
- Serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio (UPCR) are measured prior to starting therapy and periodically during treatment; **AND**

Duchenne Muscular Dystrophy (DMD) † Φ

- Patient has a confirmed mutation of the *DMD* gene that is amenable to exon 53 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] or 4-stair climb [4SC])
  - Upper limb function (ULM) test
  - North Star Ambulatory Assessment (NSAA) score
  - Forced Vital Capacity (FVC) percent predicted
- Patient had an inadequate response, or has a contraindication or intolerance, to Vitepso (viltolarsen)

† FDA Approved 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: severe hypersensitivity reactions, kidney toxicity (e.g., glomerulonephritis, persistent increase in serum cystatin C, proteinuria, 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 or more 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] or 4-stair climb [4SC], etc.)
- 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	Administer 30 mg/kg intravenously once weekly

VII. Billing Code/Availability Information

HCPCS Code:

- J1429 – Injection, golodirsen, 10 mg; 1 billable unit = 10 mg

NDC:

- Vyondys 53 100 mg/2 mL single-dose vial: 60923-0465-xx

VIII. References

1. Vyondys 53 [package insert]. Cambridge, MA; Sarepta Therapeutics, Inc.; June 2024. Accessed July 2024.
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3. 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.
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5. 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.
6. Muntoni F, Frank D, Sardone V, et al. Golodirsen Induces Exon Skipping Leading to Sarcolemmal Dystrophin Expression in Duchenne Muscular Dystrophy Patients With Mutations Amenable to Exon 53 Skipping (S22.001). Neurology Apr 2018, 90 (15 Supplement) S22.001
7. 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.
8. 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.
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10. 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.
11. Birnkrant DJ, Bushby K, Bann CM, et al. Diagnosis and management of Duchenne muscular dystrophy, part 2: respiratory, cardiac, bone health, and orthopaedic management. Lancet Neurol 2018; 17:347.
12. Servais L, Mercuri E, Straub V, et al.; SKIP-NMD Study Group. Long-Term Safety and Efficacy Data of Golodirsen in Ambulatory Patients with Duchenne Muscular Dystrophy Amenable to Exon 53 Skipping: A First-in-human, Multicenter, Two-Part, Open-Label, Phase 1/2 Trial. Nucleic Acid Ther. 2022 Feb;32(1):29-39. doi: 10.1089/nat.2021.0043. Epub 2021 Nov 17.
13. Moxley RT 3rd, 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.
14. Gloss D, Moxley RT 3rd, Ashwal S, Oskoui M. Practice guideline update summary: Corticosteroid treatment of Duchenne muscular dystrophy: Report of the Guideline Development Subcommittee of the American Academy of Neurology. Neurology. 2016 Feb 2;86(5):465-72. Doi: 10.1212/WNL.0000000000002337. Reaffirmed on January 22, 2022.

15. Darras BT, Urion DK, Ghosh PS. Dystrophinopathies. GeneReviews. www.ncbi.nlm.nih.gov/books/NBK1119/. Initial Posting: September 5, 2000; Last Revision: January 20, 2022. Accessed on July 1, 2024.

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)
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:

Vyondys53 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 Vyondys53 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.