Vyondys-53™ (golodirsen)
(Intravenous)

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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]:

– Vyondys-53 100 mg vial: 35 vials per 7 days

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

Duchenne muscular dystrophy

- 350 billable units every 7 days

III. Initial Approval Criteria [1-9]

Coverage is provided in the following conditions:

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.

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

- Patient had an inadequate response, or has a contraindication or intolerance, to viltolarsen; **AND**

Duchenne muscular dystrophy (DMD) † Φ

- **Patient is initiated on therapy between the ages of 6 to 15 years old; AND**
- **Patient able to ambulate at least 250 meters while walking independently over 6 minutes within 4 weeks of initiation of therapy; AND**
  - **Patient has a North Star Ambulatory Assessment (NSAA) total greater than 17 within 4 weeks of initiation of therapy; OR**
  - **Patient has a rise time less than 7 seconds within 4 weeks of initiation of therapy; AND**

- Patient must have 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 intolerance, 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 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); Φ 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, renal toxicity/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

- 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	Administer 30 mg/kg via intravenous infusion once weekly. – Serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio (UPCR) should be measured before starting therapy. Consider measurement of glomerular filtration rate prior to initiation of Vyondys-53.

VI. 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

VII. References

1. Vyondys 53 [package insert]. Cambridge, MA; Sarepta Therapeutics, Inc.; February 2021. Accessed July 2021.
2. 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.
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.
4. 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.
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.
9. Frank DE, Schnell FJ, Akana C, et al. Increased dystrophin production with golodirsen in patients with Duchenne muscular dystrophy. Neurology. 2020 May 26;94(21):e2270-e2282. doi: 10.1212/WNL.0000000000009233. Epub 2020 Mar 5

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

Medicare Part B Administrative Contractor (MAC) Jurisdictions		
Jurisdiction	**Applicable State/US Territory**	**Contractor**
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