MEDICA®

Vyondys 53™ (golodirsen)
(Intravenous)

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I. Length of Authorization

Coverage will be for 12 months and may be renewed.

II. Dosing Limits

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

- Vyondys 53 100 mg/2 mL single-dose vial: 35 vials per 7 days

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

- 350 billable units (3500 mg) every 7 days

III. Initial Approval Criteria

Coverage is provided in the following conditions:

Universal Criteria [1,9]

- Patient is not on concomitant therapy with other DMD-directed antisense oligonucleotides (e.g., eteplirsen, casimersen, viltolarsen, etc.); **AND**
- 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 [1-11]

- 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 intolerance, for at least 3 months; **AND**
- Patient retains meaningful voluntary motor function (e.g., patient is able to speak, manipulate objects using upper extremities, ambulate, etc.); **AND**

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- 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

IV. Renewal Criteria [1]

Coverage may be renewed based upon the following criteria:

- Patient 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: severe hypersensitivity reactions, kidney toxicity (e.g., fatal glomerulonephritis, persistent increase in serum cystatin C, proteinuria, 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
  - For all of the below, comparison would be to historical controls
    - 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 ULM test
    - Stability, improvement, or slowed rate of decline in NSAA
    - Stability, improvement, or slowed rate of decline in FVC% predicted
    - Improvement in quality of life

V. Dosage/Administration [1]

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

VI. Billing Code/Availability Information

HCPCS Code:

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- 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

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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)

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: https://www.cms.gov/medicare-coverage-database/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
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

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