Vyondys-53™ (golodirsen)
(Intravenous)

Effective Date: 03/06/2020
Review Date: 03/06/2020, 11/02/2020, 7/15/2021
Revision date: 03/06/2020, 11/02/2020, 7/15/2021
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 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

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., eteplirsen, casimersen, viltolarsen, etc.); AND

- For patient’s new to therapy they must have an inadequate response, or have a contraindication or intolerance, to viltolarsen; AND

Duchenne muscular dystrophy (DMD) † Φ

- 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 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 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
  - Upper limb function (ULM) test
  - 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**

- 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
  - 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**

<table>
<thead>
<tr>
<th>Indication</th>
<th>Dose</th>
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<tbody>
<tr>
<td>Duchenne muscular dystrophy</td>
<td>Administer 30 mg/kg via intravenous infusion once weekly.</td>
</tr>
<tr>
<td></td>
<td>- Measure glomerular filtration rate prior to initiation.</td>
</tr>
</tbody>
</table>

Store refrigerated at 2°C to 8°C. Do not freeze. Protect from light.
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

Appendix 1 – Covered Diagnosis Codes

<table>
<thead>
<tr>
<th>ICD-10</th>
<th>ICD-10 Description</th>
</tr>
</thead>
<tbody>
<tr>
<td>G71.01</td>
<td>Duchenne or Becker muscular dystrophy</td>
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</table>

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](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

<table>
<thead>
<tr>
<th>Jurisdiction</th>
<th>Applicable State/US Territory</th>
<th>Contractor</th>
</tr>
</thead>
<tbody>
<tr>
<td>E (1)</td>
<td>CA, HI, NV, AS, GU, CNMI</td>
<td>Noridian Healthcare Solutions, LLC</td>
</tr>
<tr>
<td>F (2 &amp; 3)</td>
<td>AK, WA, OR, ID, ND, SD, MT, WY, UT, AZ</td>
<td>Noridian Healthcare Solutions, LLC</td>
</tr>
<tr>
<td>5</td>
<td>KS, NE, IA, MO</td>
<td>Wisconsin Physicians Service Insurance Corp (WPS)</td>
</tr>
<tr>
<td>6</td>
<td>MN, WI, IL</td>
<td>National Government Services, Inc. (NGS)</td>
</tr>
<tr>
<td>H (4 &amp; 7)</td>
<td>LA, AR, MS, TX, OK, CO, NM</td>
<td>Novitas Solutions, Inc.</td>
</tr>
<tr>
<td>8</td>
<td>MI, IN</td>
<td>Wisconsin Physicians Service Insurance Corp (WPS)</td>
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<tr>
<td>N (9)</td>
<td>FL, PR, VI</td>
<td>First Coast Service Options, Inc.</td>
</tr>
<tr>
<td>J (10)</td>
<td>TN, GA, AL</td>
<td>Palmetto GBA, LLC</td>
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<tr>
<td>M (11)</td>
<td>NC, SC, WV, VA (excluding below)</td>
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</tr>
<tr>
<td>L (12)</td>
<td>DE, MD, PA, NJ, DC (includes Arlington &amp; Fairfax counties and the city of Alexandria in VA)</td>
<td>Novitas Solutions, Inc.</td>
</tr>
<tr>
<td>K (13 &amp; 14)</td>
<td>NY, CT, MA, RI, VT, ME, NH</td>
<td>National Government Services, Inc. (NGS)</td>
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<tr>
<td>15</td>
<td>KY, OH</td>
<td>CGS Administrators, LLC</td>
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