Prior Authorization Review Panel

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CHC-MCO Policy Submission

A separate copy of this form must accompany each policy submitted for review.
Policies submitted without this form will not be considered for review.

Plan: PA Health & Wellness	**Submission Date: 02/01/2022**
Policy Number: PA.CP.PHAR.453	**Effective Date: 01/2020** **Revision Date: 01/2022**
Policy Name: Golodirsen (Vyondys 53)	

Type of Submission – Check all that apply:

- ☐ **New Policy**
- ✓ **Revised Policy***
- ☐ **Annual Review - No Revisions**
- ☐ **Statewide PDL -** *Select this box when submitting policies for Statewide PDL implementation and when submitting policies for drug classes included on the Statewide PDL.*

***All revisions to the policy must be highlighted using track changes throughout the document.**

Please provide any changes or clarifying information for the policy below:

1Q 2022 annual review: added Amondys 45 to examples of exon-skipping therapies; references reviewed and updated.

Name of Authorized Individual (Please type or print): **Venkateswara R. Davuluri, MD**	**Signature of Authorized Individual:**

Clinical Policy: Golodirsen (Vyondys 53)

Reference Number: PA.CP.PHAR.453
Effective Date: 01/2020
Last Review Date: 01/2022

Revision Log

Description
Golodirsen (Vyondys 53™) is an antisense oligonucleotide.

FDA Approved Indication(s)
Vyondys 53 is indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the DMD gene that is amenable to exon 53 skipping.

Limitation(s) of use: This indication is approved under accelerated approval based on an increase in dystrophin production in skeletal muscle observed in patients treated with Vyondys 53. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

Policy/Criteria
Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.

I. Requirements for Prior Authorization of Vyondys 53 (golodirsen)

A. Prescriptions That Require Prior Authorization

All prescriptions for Vyondys 53 (golodirsen) must be prior authorized.

B. Review of Documentation for Medical Necessity

In evaluating a request for prior authorization of a prescription for Vyondys 53 (golodirsen), the determination of whether the requested prescription is medically necessary will take into account whether:

1. The beneficiary has a diagnosis that is:
   a. Indicated in the FDA-approved package insert, **OR**

   b. Listed in nationally recognized compendia for the determination of medically-accepted indications for off-label uses for Vyondys 53 (golodirsen)

AND

2. Vyondys 53 (golodirsen) is prescribed by or in consultation with a neurologist with experience treating Duchenne muscular dystrophy

AND

3. The beneficiary has documentation of a baseline evaluation, including a standardized assessment of motor function, by a neurologist with experience treating Duchenne muscular dystrophy

AND

4. The beneficiary will receive concurrent corticosteroids unless contraindicated or intolerant

AND

5. Vyondys 53 is not prescribed concurrently with other exon-skipping therapies (e.g., Amondys 45, Exondys 51, Viltepso)

OR

6. The beneficiary does not meet the clinical review guidelines listed above, but, in the professional judgment of the physician reviewer, the services are medically necessary to meet the medical needs of the beneficiary.

FOR RENEWALS OF PRESCRIPTIONS FOR Vyondys 53 (golodirsen) - The determination of medical necessity of requests for prior authorizationof renewals of prescriptions for Vyondys 53 (golodirsen), that were previously approved, will take into account whether:

1. Vyondys 53 (golodirsen) is prescribed by or in consultation with a neurologist with experience treating Duchenne muscular dystrophy

AND

2. The beneficiary has documentation of an annual evaluation, including an assessment of motor function ability, by a neurologist with experience treating Duchenne muscular dystrophy

AND

3. Based on the prescriber's assessment, the beneficiary continues to benefit from Vyondys 53 (golodirsen)

AND

4. The beneficiary will receive concurrent corticosteroids unless contraindicated or intolerant

AND

5. Vyondys 53 is not prescribed concurrently with other exon-skipping therapies (e.g., Amondys 45, Exondys 51, Viltepso)

OR

6. The beneficiary does not meet the clinical review guidelines listed above, but, in the professional judgment of the physician reviewer, the services are medically necessary to meet the medical needs of the beneficiary.

II. Clinical Review Process

Prior authorization personnel will review the request for prior authorization and apply the clinical guidelines in Section I.B. above, to assess the medical necessity of the request for a prescription for Vyondys 53 (golodirsen). If the guidelines in Section I.B are met, the reviewer will prior authorize the prescription. If the guidelines are not met, the prior authorization request will be referred to a physician reviewer for a medical necessity determination. Such a request for prior authorization will be approved when, in the professional judgment of the physician reviewer, the services are medically necessary to meet the medical needs of the beneficiary.

III. Approval Duration: 6 months

IV. Appendices/General Information

Appendix A: Abbreviation/Acronym Key

6MWT: 6-minute walk test
DMD: Duchenne muscular dystrophy
FDA: Food and Drug Administration
FVC: forced vital capacity
ICER: Institute for Clinical and Economic Review
LVEF: left ventricular ejection fraction

Appendix B: Therapeutic Alternatives

This table provides a listing of preferred alternative therapy recommended in the approval criteria. The drugs listed here may not be a formulary agent for all relevant lines of business and may require prior authorization.

Drug Name	Dosing Regimen	Dose Limit/ Maximum Dose
prednisone*	0.3-0.75 mg/kg/day or 10 mg/kg/weekend PO	Based on weight
Emflaza™ (deflazacort)	0.9 mg/kg/day orally once daily	Based on weight

Therapeutic alternatives are listed as Brand name® (generic) when the drug is available by brand name only and generic (Brand name®) when the drug is available by both brand and generic.
**Off-label*

Appendix C: Contraindications/Boxed Warnings

None reported

Appendix D: General Information

- Corticosteroids are routinely used in DMD management with established efficacy in slowing decline of muscle strength and function (including motor, respiratory, and cardiac). They are recommended for all DMD patients per the American Academy of Neurology (AAN) and DMD Care Considerations Working Group; in addition, the AAN guidelines have been endorsed by the American Academy of Pediatrics, the American Association of Neuromuscular & Electrodiagnostic Medicine, and the Child Neurology Society.
  - The DMD Care Considerations Working Group guidelines, which were updated in 2018, continue to recommend corticosteroids as the mainstay of therapy.
  - In an evidence report published August 2019, the Institute for Clinical and Economic Review (ICER) states that current evidence is insufficient to conclude that Vyondys 53 has net clinical benefit when added to corticosteroids and supportive care versus corticosteroids and supportive care alone.
- Prednisone is the corticosteroid with the most available evidence. A second corticosteroid commonly used is deflazacort, which was FDA approved for DMD in February 2017.
- The inclusion criteria for Study 4053-US-101 (NCT02310906) used to support the FDA approval of Vyondys 53 enrolled male patients age 6-15 years old with a mean 6MWT distance of 250 m or more at screening and baseline visits, LVEF ≥ 50% based on screening echocardiogram (ECHO), and stable pulmonary function with FVC ≥ 50%.

V. Dosage and Administration

Indication	Dosing Regimen	Maximum Dose
DMD	30 mg/kg IV once weekly	30 mg/kg

VI. Product Availability

Single-dose vial for injection: 100 mg/2 mL (50 mg/mL)

VII. References

1. Vyondys 53 Prescribing Information. Cambridge, MA: Sarepta Therapeutics, Inc.; February 2021. Available at: https://www.vyondys53.com. Accessed September 14, 2021.
2. 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; 9(1): 77-93.
3. Gloss D, Moxley RT, Ashwal S, Oskoui M. Practice guideline update summary: corticosteroid treatment of Duchenne muscular dystrophy. Neurology. 2016; 86: 465-472. Reaffirmed on January 26, 2019.
4. Frank D, Mercuri E, Servais L, et al. Golodirsen induces exon skipping leading to sarcolemmal dystrophin expression in patients with genetic mutations amenable to exon 53 skipping. Paper presented at: Annual Clinical Genetics Meeting of the American College of Medical Genetics and Genomics; April 2-6, 2019; Seattle, WA.
5. Chamberlain JS. Dystrophin levels required for genetic correction of Duchenne muscular dystrophy. Basic Apply. Myol. 1997; 7(3&4): 251-255.
6. Neri M, Torelli S, Brown S, et al. Dystrophin levels as low as 30% are sufficient to avoid muscular dystrophy in the human. Neuromuscul Disord. 2007; doi:10.1016/j.nmd.2007.07.005.

7. Institute for Clinical and Economic Review. Deflazacort, eteplirsen, and golodirsen for Duchenne muscular dystrophy: Effectiveness and value. Published August 15, 2019. Available at: https://icer-review.org/material/dmd-final-evidence-report. Accessed September 14, 2021.
8. Vyondys 53 Formulary Submission Dossier V1.0. Sarepta Therapeutics, Inc, December 2019.
9. 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: 251-267.

Coding Implications
Codes referenced in this clinical policy are for informational purposes only. Inclusion or exclusion of any codes does not guarantee coverage. Providers should reference the most up-to-date sources of professional coding guidance prior to the submission of claims for reimbursement of covered services.

HCPCS Codes	Description
J1429	Injection, golodirsen, 10 mg

Reviews, Revisions, and Approvals	Date
Policy created	01/2020
1Q 2021 annual review: added requirement that member not received concurrent Viltepso; references reviewed and updated.	01/2021
1Q 2022 annual review: added Amondys 45 to examples of exon-skipping therapies; references reviewed and updated.	01/2022