Reference number(s)
4818-A

STANDARD MEDICARE PART B MANAGEMENT

VYONDYS 53 (golodirsen)

POLICY

I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indication
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.

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.

All other indications will be assessed on an individual basis. Submissions for indications other than those enumerated in this policy should be accompanied by supporting evidence from Medicare approved compendia.

II. DOCUMENTATION

The following documentation must be available, upon request, for all submissions:
A. Initial requests: laboratory confirmation of Duchenne muscular dystrophy (DMD) diagnosis with a *DMD* gene mutation that is amenable to exon 53 skipping (refer to examples in Appendix)
B. Continuation of therapy requests: documentation (e.g., chart notes) of response to therapy

III. PRESCRIBER SPECIALTIES

This medication must be prescribed by or in consultation with a physician who specializes in the treatment of Duchenne muscular dystrophy.

IV. CRITERIA FOR INITIAL APPROVAL

Duchenne Muscular Dystrophy
Authorization of 6 months may be granted for treatment of DMD when all of the following criteria are met:
A. Genetic testing was conducted to confirm the diagnosis of DMD and to identify the specific type of *DMD* gene mutation.
B. The *DMD* gene mutation is amenable to exon 53 skipping (refer to examples in appendix).

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C. Treatment with Vyondys 53 is initiated before the age of 16.
D. Member is able to achieve an average distance of at least 250 meters while walking independently over 6 minutes.
E. Member will not exceed a dose of 30 mg/kg once weekly.
F. The requested medication will be not used concomitantly with viltolarsen.

V. CONTINUATION OF THERAPY

All members (including new members) requesting authorization for continuation of therapy must be currently receiving therapy with the requested agent.

Authorization for 12 months may be granted when all of the following criteria are met:
A. The member is currently receiving therapy with Vyondys 53.
B. Vyondys 53 is being used to treat an indication enumerated in Section IV.
C. The member is receiving benefit from therapy as evidenced by remaining ambulatory (e.g., able to walk with or without assistance, not wheelchair dependent).
D. The member will not exceed a dose of 30 mg/kg once weekly.
E. Vyondys 53 will not be used concomitantly with viltolarsen.

VI. APPENDIX

Examples of DMD gene mutations (exon deletions) amenable to exon 53 skipping
1. Deletion of exon 52
2. Deletion of exon 45-52
3. Deletion of exon 47-52
4. Deletion of exon 48-52
5. Deletion of exon 49-52
6. Deletion of exon 50-52

VII. REFERENCES

1. Vyondys 53 [package insert]. Cambridge, MA: Sareta Therapeutics; February 2021.
2. Watanabe N, Nagata T, Satou Y, et al. NS-065/NCNP-01: An Antisense Oligonucleotide for Potential Treatment of Exon 53 Skipping in Duchenne Muscular Dystrophy. *Mol Ther Nucleic Acids*. 2018;13:442–449. doi:10.1016/j.omtn.2018.09.017
3. Vyondys 53™ (golodirsen) eDossier. AMCP Formulary Decisions. AmerisourceBergen Corporation. Conshohocken, PA. Available at: www.formularydecisions.com. Accessed April 15, 2020.

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