STATE OF WEST VIRGINIA
DEPARTMENT OF HUMAN RESOURCES
BUREAU FOR MEDICAL SERVICES

Office of Pharmacy Services
Prior Authorization Criteria

VYONDYS 53® (golodirsen)

May be billed as a Medical ("Buy & Bill") claim under J1429 –
contact Acentra: P (304) 343-9663/ F (866) 209-9632
or
May be billed as a Pharmacy Point-of-Sale (POS) claim –
contact Rational Drug Therapy Program: P (800) 847-3859/ F (800) 531-7787

VYONDYS 53 is an antisense oligonucleotide 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.

A clinical benefit of VYONDYS 53 has not been established. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

Initial authorization requires review by the Medical Director and may be approved when all of the following criteria is met:

1. Must be prescribed by a Neurologist or from a physician who has provided documentation of a formal consultation with a neurologist; **AND**

2. Patient must have a confirmed mutation of a DMD gene that is amenable to exon 53 skipping (chart notes required); **AND**

3. The patient must meet all label requirements as recommended by the FDA and the manufacturer; **AND**

4. Baseline renal function must be evaluated, and documentation provided with the request for Vyondys 53; **AND**

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5. **Patient must be stabilized on corticosteroid therapy for at least 6 months prior to the request for coverage of Vyondys 53**. Documentation must be supplied detailing the prescribed steroid therapy and the patient must continue this therapy while receiving Vyondys 53.
   NOTE: If the patient cannot take steroid therapy, clinical justification must be provided by the physician, otherwise, the prior authorization request shall be immediately denied; **AND**

6. The results of appropriate and validated baseline functional tests must be submitted with the initial request for therapy. These results will be considered valid only if taken after the patient has received corticosteroid therapy for at least 6 months.
   Acceptable tests may include, **but are not limited to**, any of the following:
   a. Ambulatory patients: Six-Minute Walk Test (6MWDT) distance of > 180 meters is required for approval.
   b. Non-ambulatory patients: Brooke Upper Extremity Function Scale of 5 or less **AND** a Forced Vital Capacity (FVC) of ≥ 30% of predicted value are required for approval.

   Other functional assessment tests may be accepted on a case-by-case basis at the discretion of the Medical Director. These tests must be quantitative in nature and accompanied with supporting documentation and references describing the test.

Initial authorization approval is limited to 6 months at a time.
Criteria for Continuation Approval requires the following conditions to be met:

1. Follow-up functional test results must show stabilization or improvement of patient function compared to baseline measures; **AND**
2. The results of regular renal function tests (as recommended by the manufacturer*) must be supplied with every request for Vyondys 53; **AND**
3. Patient must maintain 100% compliance on all scheduled therapy – Vyondys 53 must be dosed once per week and maintenance steroid therapy must continue as prescribed by the physician. Failure to maintain compliance with prescribed therapy shall result in immediate discontinuation of coverage unless the disruption can be medically justified by the prescribing physician.

* Measurement of glomerular filtration rate (GFR) by 24-hour urine collection prior to initiation of therapy is recommended. Monthly monitoring for proteinuria by dipstick urinalysis and monitoring of serum cystatin C every three months is recommended. In the case of a confirmed dipstick proteinuria of 2+ or greater or elevated serum cystatin C, a 24-hour urine collection to quantify proteinuria and assess GFR should be performed.

References:

1. Vyondys 53 [package insert]. Cambridge, MA: Sarepta Therapeutics; 2021. https://www.vyondys53.com/pi (Accessed 11/16/2023)
2. Lexicomp monograph for Vyondys 53 – Reviewed 11/16/2023
3. Drug Trials Snapshots: Vyondys 53 (https://www.fda.gov/drugs/drug-approvals-anddatabases/drug-trials-snapshots-vyondys-53) (Accessed 11/22/2023)
4. Measures of Clinical Assessment in Patients with Duchenne Muscular Dystrophy (DMD) https://doi.org/10.1186/s12014-016-9109-x (Accessed 11/22/2023)
5. Birnrant et al. Lancet Neurol. 2018 March; 17(3): 251-267. Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and neuromuscular, rehabilitation, endocrine, and gastrointestinal and nutritional management (Accessed 11/22/2023)

Please refer to the member's contract benefits in effect at the time of service to determine coverage or non-coverage of these services as it applies to an individual member according to BMS coverage and policy guidelines.