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

Office of Pharmacy Service
Prior Authorization Criteria

VYONDYS 53® (golodirsen)
Effective 02/19/2020

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.

VYONDYS 53 is currently billable only as a Pharmacy Point-of-Sale (POS) claim:

- Pharmacy POS - Contact RDTP, tel: (800) 847-3859/ fax (800) 531-7787

All requests require review by the Medical Director and may be approvable once the following criteria are met:

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

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

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

4. Request must either be from a neurologist or from a physician who has provided documentation of a formal consultation with a neurologist; **AND**

   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

5. 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) (patient must achieve > 180 meters for approval.

b. Non-ambulatory patients: Brooke Upper Extremity Function Scale (of 5 or less) **AND** a Forced Vital Capacity 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.

All prior authorization approvals are limited to 6 months at time and continuation of coverage 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 (Sarepta Therapeutics) – Revised 12/2019
2.) Lexicomp monograph for Vyondys 53 – reviewed 2/19/2020
3.) Drug Trials Snapshots: Vyondys 53 (https://www.fda.gov/drugs/drug-approvals-and-databases/drug-trials-snapshots-vyondys-53)
4.) Measures of Clinical Assessment in Patients with Duchenne Muscular Dystrophy (DMD) https://sarepta.appdataroom.com/download/v/bd16dff387135c4643386085c1bdcbc58fc508ef490459?ts=1582134595&sig=35e5f191eed1241b58063411fc49504f65bf63e7&trackingGuid=CDB061BF-A492-4090-A22E-2FADB6F9B100&groupId=4411
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