PHARMACY POLICY STATEMENT
TRICARE

DRUG NAME	Vyondys 53 (golodirsen)
BENEFIT TYPE	Medical
SITE OF SERVICE ALLOWED	Home/Office/Outpatient
STATUS	Prior Authorization Required

Vyondys 53 is an antisense oligonucleotide initially approved by the FDA in 2019. It 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

Vyondys 53 (golodirsen) will be considered for coverage when the following criteria are met:

DUCHENNE MUSCULAR DYSTROPHY (DMD)

For **initial** authorization:

1. Member has a diagnosis of DMD with confirmed mutation of DMD gene that is amenable to exon 53 skipping (genetic testing results required); AND
2. Medication is being prescribed by or in consultation with a DMD specialist (i.e., neurologist); AND
3. Member is currently stable on corticosteroid for at least 6 months prior to starting therapy, unless not tolerated or contraindicated; AND
4. Member has had a 90-day trial and failure of, or contraindication to Viltepso; AND
5. Member's body weight within the last 30 days has been submitted.
6. **Dosage allowed/Quantity limit:** 30 mg per kg of body weight once weekly.

If all the above requirements are met, the medication will be approved for 6 months.

For **reauthorization**:

1. Chart notes must show stability or slowed rate of decline of the member's motor function compared to baseline

If all the above requirements are met, the medication will be approved for an additional 6 months.

TRICARE Prime® Demo by CareSource Military & Veterans™ considers Vyondys 53 (golodirsen) not medically necessary for the treatment of conditions that are

not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
1/21/2020	New policy for Vyondys 53 created.
06/17/2020	Length of corticosteroid trial specified to be at least 3 months. Age requirement removed.
01/14/2021	Added prescriber requirement. Simplified ambulatory requirement. Added requirement of stability or slowed rate of decline of motor function in reauth section. Added a trial of Viltepso.
04/06/2021	Increased duration of steroid trial to 6 months.
03/02/2022	Transferred to new template. Added weight requirement to ensure appropriate dosing. Removed ambulatory requirement.
3/31/2023	Removed ambulatory requirement from initial criteria.

References:

1. Vyondys 53 [Package Insert]. Cambridge, MA: Sarepta Therapeutics, Inc.; February 2021.
2. Sarepta Therapeutics, Inc. Phase I/II Study of SRP-4053 in DMD Patients. NLM Identifier: NCT02310906.
3. 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 [published correction appears in Lancet Neurol. 2018 Apr 4:]. *Lancet Neurol*. 2018;17(3):251-267.
4. Gloss D, Moxley RT 3rd, Ashwal S, Oskoui M. Practice guideline update summary: Corticosteroid treatment of Duchenne muscular dystrophy: Report of the Guideline Development Subcommittee of the American Academy of Neurology. *Neurology*. 2016;86(5):465-472.
5. Frank DE, Schnell FJ, Akana C, et al. Increased dystrophin production with golodirsen in patients with Duchenne muscular dystrophy. *Neurology*. 2020;94(21):e2270-e2282. doi:10.1212/WNL.0000000000009233

Effective date: 01/01/2026
Revised date: 03/31/2023