golodirsen (Vyondys 53)

Commercial Medical Benefit Drug Policy

Place of Service
Infusion Center Administration
Home Infusion Administration
Hospital Outpatient Facility Administration
Office Administration
Outpatient Facility Infusion Administration

Drug Details

USP Category: GENETIC OR ENZYME OR PROTEIN DISORDER: REPLACEMENT, MODIFIERS, TREATMENT
Mechanism of Action: Antisense oligonucleotide that binds to exon 53 of dystrophin pre-mRNA resulting in exon exclusion.
HCPCS:
J1429:Injection, golodirsen, 10 mg
How Supplied:
100 mg/2 mL (single-dose vial)

Condition(s) listed in policy *(see coverage criteria for details)*

- Duchenne Muscular Dystrophy (DMD)

Any condition not listed in this policy requires a review to confirm it is medically necessary. For conditions that have not been approved for intended use by the Food and Drug Administration (i.e., off-label use), the criteria outlined in the Health and Safety Code section 1367.21 must be met.

Special Instructions and Pertinent Information

Provider must submit documentation (such as office chart notes, lab results or other clinical information) to ensure the member has met all medical necessity requirements.

The member's specific benefit may impact drug coverage. Other utilization management processes, and/or legal restrictions may take precedence over the application of this clinical criteria.

Members with the following plans: **PPO, Direct Contract HMO, and when applicable, ASO, Shared Advantage, HMO (non-direct)** may be required to have their medication administered at a preferred site of service, including the home, a physician's office, or an independent infusion center not associated with a hospital.

For members that cannot receive infusions in the preferred home or ambulatory setting AND meet one of the following criteria points, drug administration may be performed at a hospital outpatient facility infusion center.

CRITERIA FOR HOSPITAL OUTPATIENT FACILITY ADMINISTRATION

MCG Care Guidelines, 19th edition, 2015

ADMINISTRATION OF THIS DRUG IN THE HOSPITAL OUTPATIENT FACILITY SITE OF CARE REQUIRES ONE OF THE FOLLOWING: (*Supporting Documentation must be submitted)*

1. Patient is initiating on Vyondys 53 (allowed for first 4 infusions) or is being re-initiated on Vyondys 53 after at least 6 months off therapy. *Subsequent doses will require medical necessity for continued use in the hospital outpatient facility site of care.*

OR

Additional clinical monitoring is required during administration as evidenced by one of the following:

2. Patient has experienced a previous severe adverse event on Vyondys 53 based on documentation submitted.
3. Patient continues to experience moderate to severe adverse events on Vyondys 53 based on documentation submitted, despite receiving premedication such as acetaminophen, steroids, diphenhydramine, fluids, etc.
4. Patient is clinically unstable based on documentation submitted.
5. Patient is physically or cognitively unstable based on documentation submitted.

Coverage Criteria

The following condition(s) require Prior Authorization/Preservice.

Duchenne Muscular Dystrophy (DMD)

Meets medical necessity if all the following are met:

1. Prescribed by or in consultation with a pediatric neurologist or neuromuscular specialist
2. Diagnosis of DMD that is amenable to exon 53 skipping confirmed by genetic testing
3. ***Effective 8/1/2025 and after:*** Patient is ambulatory with a baseline six-minute walk test (6MWT) ≥ 250 meters
4. ***Effective 8/1/2025 and after:*** Patient is not currently on other DMD antisense oligonucleotides (e.g. casimersen, eteplirsen, or viltolarsen)

Covered Doses:

30 mg/kg given intravenously once weekly

Coverage Period:

Through 7/31/2025: Indefinitely

Effective 8/1/2025 and after:

Initial: One year

Reauthorization: One year if ALL the below are met

1. Prescribed by or in consultation with a neurologist or neuromuscular specialist
2. Patient remains ambulatory
3. Patient has shown improvement, stable disease, or slowing of disease progression
4. Patient is not currently on other DMD antisense oligonucleotides (e.g. casimersen, eteplirsen, or viltolarsen)

ICD-10:
G71.01

References

1. AHFS. Available by subscription at http://www.lexi.com
2. DrugDex. Available by subscription at http://www.micromedexsolutions.com/home/dispatch
3. Vyondys 53 (golodirsen) Prescribing Information. Cambridge, MA: Sarepta Therapeutics Inc.; 6/2024.

Review History

Date of Last Annual Review: 2Q2025
Changes from previous policy version:

- Duchenne Muscular Dystrophy: ***Effective 8/1/2025 and after***, will add requirement for baseline ambulation, clarify combination use is not with other agents used for DMD, and add reauthorization requirements for prescriber specialty, ambulation, and clinical response (Rationale: Vyondys 53 prescribing information)

Blue Shield of California Medication Policy to Determine Medical Necessity
Reviewed by P&T Committee