blue california
Promise Health Plan

golodirsen (Vyondys 53)

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 California Code of Regulations (CCR), Title 22, Section 51303 and 51313 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.

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