

Division: Pharmacy Policy	Subject: Prior Authorization Criteria
Original Development Date: Original Effective Date: Revision Date:	January 2, 2020 March 9, 2021, October 4, 2021, March 31, 2022

## **VYONDYS 53 (golodirsen)**

**LENGTH OF AUTHORIZATION:** SIX MONTHS

**REVIEW CRITERIA:**

- Patient must have the diagnosis of Duchenne Muscular Dystrophy (DMD).
- Submission of medical records including the following:
  - Genetic testing confirming the patient has a mutation of the DMD gene that is amenable to exon 53 skipping.
  - Baseline renal function tests (i.e., glomerular filtration rate GFR).
- Medication is prescribed by or in consultation with a neurologist or a physician who specializes in treatment of DMD (i.e., pediatric neurologist, cardiologist or pulmonary specialist).
- **Patient has been on stable dose of oral corticosteroids for at least 24 weeks prior to starting therapy unless contraindicated or intolerant.**
- Patient is not concurrently treated with other DMD antisense oligonucleotides (e.g., Casimersen, Viltolarsen, or Eteplirsen).
- If the patient is ambulatory, functional level determination of baseline assessment of ambulatory function (six-minute walk test (6MWT), time to run/walk 10-meter test (TTRW), time to climb 4-stair test (TTCLIMB), time to stand (TTSTAND) or North Star Ambulatory Assessment (NSAA)) is required.
- If not ambulatory, patient must have a Brooke Upper Extremity Function Scale of five or less documented OR a Forced Vital Capacity of 30% or more.

**CONTINUATION OF THERAPY:**

- Patient met initial review criteria.
- Documentation of improvement, maintenance, or slowing of disease progression:
  - For ambulatory patients- Submission of 6MWT, TTRW, TTCLIMB, TTSTAND or NSAA.
  - For non-ambulatory patients- Submission of Brooke Upper Extremity Function Scale (five or less) documented OR a Forced Vital Capacity documented (30% or more).
- Submission of renal function tests (i.e., GFR).
- Patient is not concurrently treated with other DMD antisense oligonucleotides (e.g., Casimersen, Viltolarsen, or Eteplirsen).

**DOSING AND ADMINISTRATION:**

- Refer to product labeling at <https://www.accessdata.fda.gov/scripts/cder/daf/>
- Available as 100 mg/2 mL single-dose vial

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