| Division: Pharmacy Policy | Subject: State of Florida's Agency for Health Care Administration's Prior Authorization Criteria |
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| Original Development Date:<br>Original Effective Date:<br>Revision Date: | May 3, 2017<br><br>August 28, 2017, January 18, 2019, March 9, 2021, October 4, 2021, March 31, 2022 |

# EXONDYS 51 (eteplirsen)

**LENGTH OF AUTHORIZATION**: SIX MONTHS

**REVIEW CRITERIA**:

- Patient must have the diagnosis of Duchenne muscular dystrophy (DMD).
- Submission of medical records (e.g., chart notes, laboratory values) as genetic test is required to confirm that a patient's mutation of the DMD gene is amenable to exon 51 skipping.
- 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 is not concurrently treated with other DMD antisense oligonucleotides (e.g., Casimersen, Viltolarsen, or Golodirsen).
- Patient has been on stable dose of oral corticosteroids for at least 24 weeks prior to starting therapy unless contraindicated or intolerant.
- 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).
- Patient is not concurrently treated with other DMD antisense oligonucleotides (e.g., Casimersen, Viltolarsen, or Golodirsen).

**DOSING AND ADMINISTRATION:**

- Refer to product labeling at https://www.accessdata.fda.gov/scripts/cder/daf/
- Available as 100 mg/2 mL and 500 mg/10 mL single-dose vials