EXON SKIPPING DRUGS FOR DUCHENNE MUSCULAR DYSTROPHY

- **AMONDYS 45™/ CASIMERSEN (EXON 45)**
- **EXONDYS 51™/ ETEPLIRSEN (EXON 51)**
- **VYONDYS 53™/ GOLODIRSEN (EXON 53)**
- **VILTEPSO™/ VILTOLARSEN (EXON 53)**

Drug Class: Antisense Oligonucleotides

FDA-approved uses: Duchenne Muscular Dystrophy (DMD) with deletion amenable to prescribed drug

Available dosage forms: Weight-based clinician-administered infusion.

Coverage Criteria/Limitations for initial authorization:

- ❒ **Diagnoses**: G71.01 – FDA approved for Duchenne Muscular Dystrophy only
- ❒ **Duration of approval**:
  - **Initial authorization**: 1 year
  - **Continuation of Therapy**: 1 year
- ❒ **Prescriber Specialty**: Neurology or Physical Medicine & Rehabilitation
- ❒ **Documentation Requirements** (e.g. Labs, Medical Record, Special Studies):
  - Completed, Signed, and Dated MSA 6544-B
  - Comprehensive History and Physical detailing diagnosis of DMD from Neurologist or PM&R experienced in DMD, completed within 6 months of request.
  - Genetics testing report confirming mutation of DMD gene amenable to appropriate exon skipping therapy.
  - Documentation of being on stable dose of corticosteroid for at least 6 months prior to request, or an explanation why corticosteroids cannot be utilized.
  - Pulmonary Medicine report completed within 6 months of request, including:
    - The type of ventilatory support and the hours per day the patient is on ventilatory support, with change from previous ventilatory status
    - OR, attestation from the treating Neurologist or PM&R stating that a Pulmonary consult is unnecessary at the time of request.
  - Cardiology status/report, if pertinent
  - Monitor baseline lab work specific to each drug, according to FDA criteria
  - Baseline functional motor exam appropriate for age, performed by a Physical Therapist (PT), Neurologist, or PM&R experienced in treating DMD
- ❒ **Dosage:** Weight-based, in accordance with FDA dosage recommendations
- ❒ **Age**: In accordance with current FDA guidelines
- ❒ **Route of Administration**: Infusion
- ❒ **Place of Service**: Initial doses must be given in a clinical setting for the first 2 weeks. If the patient tolerates the infusion with no adverse effects, the drug may be given as a home-infusion.

Criteria for continuation of therapy:

- ❒ **Duration of Approval:** 1 year
- ❒ **Documentation Requirements (e.g. Labs, Medical Record, Special Studies)**:
  - Completed, Signed, and Dated MSA 6544-B
  - Comprehensive progress note by the prescribing Neurologist or PM&R detailing patient's condition, conducted since the previous authorization
    - At least 1 Neurologist/ PM&R visit within 6 months of continuation requests
  - Pulmonary Medicine Report that indicates whether the patient is on ventilation, the type of ventilation, and how many hours per day the patient is on invasive and non-invasive ventilation
    - OR, attestation from the treating Neurologist or PM&R stating that a Pulmonary consult is unnecessary at time of request
  - Monitor baseline lab work specific to each drug, according to FDA criteria Functional
  - Motor Exam by a Physical Therapist, Neurologist, or PM&R experienced in treating SMA and conducted since the previous authorization
    - For continuation authorization, MDHHS requires improvement, stabilization, or a decrease in expected loss of function (as indicated by the individual's past medical history)

Contraindications/Exclusions/Discontinuation:

- ❒ Anaphylaxis
- ❒ Compromised renal function
- ❒ Permanent 24-hour ventilator dependency

Other special considerations:

If utilizing a Specialty Pharmacy to acquire drug:

- ❒ After receiving Prior Authorization (PA) from MDHHS, Provider must fax the Approval letter and prescription to the Specialty Pharmacy. The Specialty Pharmacy will submit the claim as a Pharmacy Claim (NDC code) to the MDHHS PBM vendor.
- ❒ If patient is enrolled fee-for-service Medicaid/CSHCS, procedure codes 96365, 96366, 96367 96368, 96369, 96370, 96371, 96401, 96409, 96411, 96413, 96415, 96416, 96417 do not require PA.
- ❒ If patient is enrolled in a Medicaid and/or CSHCS managed care plan, fee-for-service provides coverage for the medication requested only. Refer to the managed care plan's authorization requirements for coverage of the procedures and services associated with this protocol, including PT evaluation/re-evaluation and other ancillary codes 96365, 96366, 96367 96368, 96369, 96370, 96371, 96401, 96409, 96411, 96413, 96415, 96416, 96417.