ALASKA MEDICAID
Prior Authorization Criteria

Duchenne Muscular Dystrophy (DMD) (Exondys 51, Amondys 45, Vyondys 53, Viltepso)

FDA INDICATIONS AND USAGE[1,2,3,4]

Exon skipping drugs are an antisense oligonucleotide, indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the DMD gene that is amenable to exon skipping. This indication is approved under accelerated approval based on an increase in dystrophin production in skeletal muscle observed in patients treated. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial.

APPROVAL CRITERIA[1,2,3,4,5,6,7,8,9,10]

1. Patient's age is to FDA label **AND**;
2. Prescribed by or in consultation with a neurologist that specializes in DMD **AND**;
3. Patient has the diagnosis of DMD confirmed by submission of lab testing demonstrating mutation of the dystrophin gene amenable to exon skipping of the applicable target exon (45, 51, or 53) **AND**;
4. Documentation of Serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio must be submitted prior to initiation **AND**;
5. Documented baseline muscle strength score from ONE or more of the following must be submitted:
    a. 6-minute walk test (6MWT)
    b. North Star ambulatory assessment (NSAA)
    c. Motor Function Measure (MFM)
    d. Brooke Upper Extremity Function Scale (of 5 or less) AND a Forced Vital Capacity of ≥ 30% of predicted value **AND**;
6. Documentation the patient will receive concurrent corticosteroids unless contraindicated or intolerant **AND;**
7. Patient's current weight has been submitted.

DENIAL CRITERIA [1,2,3,4]

1. Failure to meet approval criteria **OR;**
2. Concomitant use with another exon skipping medication.

CAUTIONS[1,2,3,4]

- See package inserts.
  - Exondys 51: https://www.exondys51hcp.com/sites/default/files/2020-08/EXONDYS51PI.pdf
  - Vyondys 53: https://www.vyondys53.com/static/patient/assets/Vyondys53_(golodirsen)_Prescribing_Information.pdf
  - Amondys 45: https://amondys45.com/Amondys45_(casimersen)_Prescribing_Information.pdf
  - Viltepso: https://www.viltepso.com/prescribing-information

DURATION OF APPROVAL

- Initial Approval: up to 6 months
- Reauthorization Approval: up to 12 months if the prescriber documents that the patient has shown improvement or is stable from baseline and current weight has been submitted.

QUANTITY LIMIT

- Viltepso - Max dose 80mg/kg
  - HCPCS – J1426
- Amondys 45 - Max dose 30mg/kg
  - HCPCS -J1427
- Exondys 51 – Max dose 30mg/kg
  - HCPCS – J1428
- Vyondys 53 - Max dose 30mg/kg
  - HCPCS – J1429

REFERENCES / FOOTNOTES:

1. Exondys 51 [package insert]. Sarepta Therapeutics, Inc. Cambridge, Massachusetts. January 2022.
2. Vyondys 53 [package insert]. Sarepta Therapeutics, Inc. Cambridge, Massachusetts. February 2021.
3. Amondys 45 [package insert]. Sarepta Therapeutics, Inc. Cambridge, Massachusetts. February 2021.
4. Viltepso [package insert]. NS Pharma, Inc. Paramus, NJ. March 2021.

5. Hwang J, Yokota T. Recent advancements in exon-skipping therapies using antisense oligonucleotides and genome editing for the treatment of various muscular dystrophies. Expert Rev Mol Med. 2019;21:e5.
6. Kole R, Krieg AM. Exon skipping therapy for Duchenne muscular dystrophy. Adv Drug Deliv Rev. 2015;87:104-107.
7. Clemens PR, Rao VK, Connolly AM, et al. Safety, tolerability, and efficacy of viltolarsen in boys with Duchenne muscular dystrophy amendable to Exon 53 skipping: A phase 2 randomized clinical trial. JAMA Neurol. 2020;77(8);1-10.
8. Alfano LN, Charleston JS, Connolly AM, et al. Long-term treatment with eteplirsen in nonambulatory patients with Duchenne muscular dystrophy. Medicine (Baltimore). 2019;98(26):e15858.
9. Shimizu-Motohashi Y, Murakami T, Kimura E, et al. Exon skipping for Duchenne muscular dystrophy: A systematic review and meta-analysis. Orphanet J Rare Dis. 2018;13(1):93.
10. Watanabe N, Nagata T, Satou Y, et al. NS-065/NCNP-01: An antisense oligonucleotide for potential treatment of exon 53 skipping in Duchenne muscular dystrophy. Mol Ther Nucleic Acids. 2018;13:442-449.