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Effective Date: 4/16/2020

Viltolarsen (NS-065) for intravenous infusion

FDA approval: Anticipated April 2020

HCPCS: J3490

Benefit: Medical

Policy:

Requests must be supported by submission of chart notes and patient specific documentation.

- A. Coverage of viltolarsen is considered investigational/experimental for all indications due to insufficient evidence of a clinical benefit.
 - a. BCBSM and BCN are awaiting the results of ongoing clinical trials to provide evidence of a clinical benefit.
- B. Quantity Limitations, Authorization Period and Renewal Criteria
 - a. Quantity Limit: N/A
 - b. Initial Authorization Period: N/A
 - c. Renewal Criteria: N/A

***Note: Coverage may differ for Medicare Part B members based on any applicable criteria outlined in Local Coverage Determinations (LCD) or National Coverage Determinations (NCD) as determined by Center for Medicare and Medicaid Services (CMS). See the CMS website at <http://www.cms.hhs.gov/>. Determination of coverage of Part B drugs is based on medically accepted indications which have supported citations included or approved for inclusion determined by CMS approved compendia.

Therapeutic considerations:

- A. **FDA approved indication / Diagnosis**
 - a. Indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients having a confirmed mutation of the DMD gene that is amenable to exon 53 skipping

**Please refer to most recent prescribing information.*

- B. **Background Information**
 - a. Duchenne muscular dystrophy (DMD) is a rare, life-limiting, progressive childhood disease that affects 1 in 3,500 – 5,000 live male births
 - b. It is estimated 8% of DMD patients have a mutation in exon 53

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- c. It is characterized by progressive muscle weakness and wasting due to the absence of dystrophin protein that causes degeneration of skeletal and cardiac muscle
- d. Affected individuals are unable to run and jump properly due to proximal muscle weakness of the leg and pelvic muscles
- e. DMD occurs as a result of mutations (mainly deletions) in the dystrophin gene, located on the X-chromosome, which normally functions to generate dystrophin which is a structural protein of muscle cells
- f. Mutations in dystrophin gene lead to an absence of or a defect in dystrophin protein, resulting in the progressive symptoms seen in a DMD patient
- g. Glucocorticoids along with surgical and physical therapeutic measures, are the main treatments used in DMD
- h. Treatment guidelines for DMD support the use of glucocorticoids as they are the only medication currently available to slow the decline in muscle strength and function which in turn reduces the risk of scoliosis and stabilizes pulmonary function
- i. Trials show muscle strength is improved when treated with prednisone at a dose of 0.75 mg/kg daily for up to 6 months
- j. The goal of glucocorticoids in an ambulatory patient is the preservation of ambulation and the minimization of later cardiac, respiratory, and orthopedic complications
- k. Continued treatment after the patient becomes non-ambulatory has shown reduction in the risk of progressive scoliosis and stabilization of pulmonary function tests
- l. However, it is important to note that glucocorticoids are not able to induce the production of dystrophin-like proteins and therefore do not alter or impact the underlying cause of DMD
- m. Exondys 51 and Vyondys 53, the only other FDA approved disease modifying therapies for DMD, are not currently recommended for use in the guidelines as there is a lack of sufficient evidence for treatment

C. Efficacy

**Please refer to most recent prescribing information.*

D. Medication Safety Considerations

Black Box Warning: No

**Please refer to most recent prescribing information.*

E. Dosing and administration

- a. Dosing: 80 mg/kg weekly

**Please refer to most recent prescribing information.*

F. How supplied

- a. Unknown at this time

References:

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4. Humbertclaude V, Hamroun D, Bezzou K, et al. Motor and respiratory heterogeneity in Duchenne patients: implication for clinical trials. *Eur J Paediatr Neurol*. 2012; 16(2): 149 – 160.
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8. Birnkrant DJ, Bushby K, Bann CM, et al. Diagnosis and management of duchenne muscular rehabilitation, endocrine, and gastrointestinal and nutritional management. *Lancet Neuro*. 2018 Mar; 17 (3): 251 – 67.
9. Malcolm, E. Viltolarsen (NS-065/NCNP-01). 2020 Feb 18. Available at: <https://musculardystrophynews.com/viltolarsen-ns-065-ncnp-01/>. Accessed on: February 18, 2020.
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11. Clinicaltrials.gov. A phase 3 randomized, double-blind, placebo-controlled, multi-center study to assess the efficacy and safety of viltolarsen in ambulant boys with duchenne muscular dystrophy (DMD) (NCT04060199). Available at: <https://clinicaltrials.gov/ct2/show/NCT04060199?term=NCT04060199&draw=2&rank=1>. Accessed on: February 18, 2020.
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13. Clinicaltrials.gov. Extension study of NS-065/NCNP-01 in boys with duchenne muscular dystrophy (DMD) (NCT03167255). Available at: <https://clinicaltrials.gov/ct2/show/NCT03167255?term=NS-065&cond=Duchenne+Muscular+Dystrophy&draw=2&rank=1>. Accessed on: February 18, 2020.

Policy History		
#	Date	Change Description
1.0	Effective Date: 4/16/2020	Preliminary drug review

* The prescribing information for a drug is subject to change. To ensure you are reading the most current information it is advised that you reference the most updated prescribing information by visiting the drug or manufacturer website or <http://dailymed.nlm.nih.gov/dailymed/index.cfm>.