



Nonprofit corporations and independent licensees
of the Blue Cross and Blue Shield Association

Medical benefit drug policies are a source for BCBSM and BCN medical policy information only. These documents are not to be used to determine benefits or reimbursement. Please reference the appropriate certificate or contract for benefit information. This policy may be updated and therefore subject to change.

Effective Date: 4/16/2020

Viltepso (Viltolarsen) for intravenous infusion

FDA approval: Anticipated April 2020

HCPCS: J3490, C9071

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

This policy and any information contained herein is the property of Blue Cross Blue Shield of Michigan and its subsidiaries, is strictly confidential, and its use is intended for the P&T committee, its members and BCBSM employees for the purpose of coverage determinations.

- 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:

1. Mendell JR, Shilling C, Leslie ND, et al. Evidence-based path to newborn screening for Duchenne muscular dystrophy. *Ann Neurol.* 2012; 71(3): 304 – 313.
2. Hoffman EP, Fischbeck KH, Brown RH, et al. Characterization of dystrophin in muscle-biopsy specimens from patients with Duchenne's or Becker's muscular dystrophy. *N Engl J Med.* 1988; 318(21): 1363 – 1368.
3. Koeks Z, Bladen CL, Salgado D, et al. Clinical outcomes in Duchenne muscular dystrophy: a study of 5345 patients from the TREAT-NMD DMD global database. *J Neuromuscul Dis.* 2017; 4(4): 293 – 306.

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.
5. Gloss D, Moxley RT, Ashwal S, Oskoui M. Practice guideline update summary: corticosteroid treatment of Duchenne muscular dystrophy: report of the guideline development subcommittee of the American Academy of Neurology. *Neurology.* 2018; 17(4): 347 – 361.
6. Van Putten M, Hulsker M, Young C, et al. Low dystrophin levels increase survival and improve muscle pathology and function in dystrophin/utrophin double-knockout mice. *FASBE J.* 2013; 27(6): 2484 – 2495.
7. Waldrop MA, Gumienny F, EL Husayni S, et al. Low-level dystrophin expression attenuating the dystrophinopathy phenotype. *Neuromuscular Disord.* 2018; 28(2): 116 – 121.
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.
10. Swanson J. NS pharma submits new drug application to FDA for viltolarsen to treat DMD amenable to exon 53 skipping. 2019 Oct 7. Available at: <https://strongly.mda.org/ns-pharma-submits-new-drug-application-to-fda-for-viltolarsen-to-treat-dmd-amenable-to-exon-53-skipping/>. Accessed on: February 18, 2020.
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.
12. Clinicaltrials.gov. Safety and dose finding study of NS-065/NCNP-01 in boys with duchenne muscular dystrophy (DMD) (NCT02740972). Available at: <https://clinicaltrials.gov/ct2/show/NCT02740972?term=NS-065&cond=Duchenne+Muscular+Dystrophy&rank=3>. Accessed on: February 18, 2020.
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										
1.2	Effective Date: 10/01/2020	PA added to BCBSM <table><tr><th>Line of Business</th><th>PA Required (Yes/No)</th></tr><tr><td>BCBS</td><td>Yes</td></tr><tr><td>BCN</td><td>Yes</td></tr><tr><td>MAPPO</td><td>No</td></tr><tr><td>BCNA</td><td>No</td></tr></table>	Line of Business	PA Required (Yes/No)	BCBS	Yes	BCN	Yes	MAPPO	No	BCNA	No
Line of Business	PA Required (Yes/No)											
BCBS	Yes											
BCN	Yes											
MAPPO	No											
BCNA	No											
1.1	Effective Date: 09/01/2020	PA added to BCN <table><tr><th>Line of Business</th><th>PA Required (Yes/No)</th></tr><tr><td>BCBS</td><td>No</td></tr><tr><td>BCN</td><td>Yes</td></tr><tr><td>MAPPO</td><td>No</td></tr><tr><td>BCNA</td><td>No</td></tr></table>	Line of Business	PA Required (Yes/No)	BCBS	No	BCN	Yes	MAPPO	No	BCNA	No
Line of Business	PA Required (Yes/No)											
BCBS	No											
BCN	Yes											
MAPPO	No											
BCNA	No											
1.0	Effective Date: 4/16/2020	Preliminary drug review										

This policy and any information contained herein is the property of Blue Cross Blue Shield of Michigan and its subsidiaries, is strictly confidential, and its use is intended for the P&T committee, its members and BCBSM employees for the purpose of coverage determinations.

** 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>.*

Blue Cross Blue Shield/Blue Care Network of Michigan

Medication Authorization Request Form



**Blue Cross
Blue Shield
Blue Care Network**
of Michigan

This form is to be used by participating physicians to obtain coverage for **drugs covered under the medical benefit**. For commercial members only, please complete this form and submit via fax to 1-877-325-5979. If you have any questions regarding this process, please contact BCBSM Provider Relations and Servicing or the Medical Drug Helpdesk at 1-800-437-3803 for assistance.

Nonprofit corporations and independent licensees of the Blue Cross and Blue Shield Association

PATIENT INFORMATION	PHYSICIAN INFORMATION
Name	Name
ID Number	Specialty
D.O.B. ☐ Male ☐ Female	Address
Diagnosis	City /State/Zip
Drug Name	Phone/Fax: P: () - F: () -
Dose and Quantity	NPI
Directions	Contact Person
Date of Service(s)	Contact Person Phone / Ext.

STEP 1: DISEASE STATE INFORMATION

- Is this request for: ☐ Initiation ☐ Continuation Date patient started therapy: _____
- Administered by patient or a medical professional? ☐ patient (self) ☐ health care professional (physician, nurse, etc.)
- Site of administration? ☐ Provider office/Home infusion ☐ Other: _____
☐ Hospital outpatient facility (go to #4) Reason for Hospital Outpatient administration: _____
- Please specify location of administration if hospital outpatient infusion: _____
- Please provide the NPI number for the place of administration: _____
- Initiation AND Continuation of therapy:**
 - What is the patient's diagnosis? _____
 - What other medication has the patient received for their condition? Please list _____
 - Please describe the response to previous therapies: _____
 - Will the patient be receiving any other treatment for the listed condition while on this medication? Please list: _____
 - Please list any labs values important for diagnosing or monitoring this patient's condition: _____
- Continuation of therapy:**
 - Has the patient progressed while on this medication? ☐ yes ☐ no
 - How has the patient's condition changed while on this medication?
☐ Improved: Please describe: _____
☐ Stable: please describe: _____
☐ Worsened; Please describe: _____
☐ Other; Please describe: _____

Chart notes are required for the processing of all requests. Please add any other supporting medical information necessary for our review (required)

Coverage will not be provided if the prescribing physician's signature and date are not reflected on this document.

☐ Request for expedited review: I certify that applying the standard review time frame may seriously jeopardize the life or health of the member or the member's ability to regain maximum function

Physician's Name	Physician Signature	Date
Step 2: Checklist	☐ Form Completely Filled Out ☐ Provide chart notes	☐ Attach test results
Step 3: Submit	By Fax: BCBSM Specialty Pharmacy Mailbox 1-877-325-5979	By Mail: BCBSM Specialty Pharmacy Program P.O. Box 312320, Detroit, MI 48231-2320

Confidentiality notice: This transmission contains confidential information belonging to the sender that is legally privileged. This information is intended only for use of the individual or entity named above. The authorized recipient of this information is prohibited from disclosing this information to any other party. If you are not the intended recipient, you are hereby notified that any disclosure, copying, distribution or action taken in reliance on the contents of this document is strictly prohibited. If you have received this in error, please notify the sender to arrange for the return of this document.

7/26/2018, 9/18/2018; 1/31/2020; 3/17/2020

