 Draft Revision Policy: Do Not Implement

Nusinersen (Spinraza™)

IMPORTANT REMINDER

We develop Medical Policies to provide guidance to Members and Providers. This Medical Policy relates only to the services or supplies described in it. The existence of a Medical Policy is not an authorization, certification, explanation of benefits or a contract for the service (or supply) that is referenced in the Medical Policy. For a determination of the benefits that a Member is entitled to receive under his or her health plan, the Member's health plan must be reviewed. If there is a conflict between the medical policy and a health plan or government program (e.g., TennCare), the express terms of the health plan or government program will govern.

The proposal is to add text/statements in red and to delete text/statements with strikethrough:

POLICY

INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indications

- Nusinersen for the treatment of spinal muscular atrophy (SMA) is considered **medically necessary** if the coverage criteria are met.
- Nusinersen for the treatment of other conditions/diseases is considered **investigational.**

PRESCRIBER SPECIALITIES

This medication must be prescribed by or in consultation with a neurologist or a physician who specializes in the treatment of spinal muscular atrophy.

DOCUMENTATION

- Baseline documentation of one or more of the following:
  - Motor function/milestones, including but not limited to, the following validated scales: Hammersmith Infant Neurologic Exam (HINE), Hammersmith Functional Motor Scale Expanded (HFMSE), Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND), Bayley Scales of Infant and Toddler development Third Ed. (BSID-III), 6-minute walk test (6MWT), upper limb module (ULM), motor function measure 32 (MFM32), revised upper limb module (RULM), etc.
  - Respiratory function tests [e.g., forced vital capacity (FVC), etc.]
  - Exacerbations necessitating hospitalization and/or antibiotic therapy for respiratory infection in the preceding year/timeframe
  - Patient weight (for patients without a gastrostomy tube)
- Patient must have the following laboratory tests at baseline and prior to each administration*: platelet count, prothrombin time; activated partial thromboplastin time, and quantitative spot urine protein testing.
- Documentation of a genetic test confirms no more than **3 copies** of the SMN2 gene. *Note: Patients with >3 copies of the SMN2 gene will be reviewed on a case-by-case basis*)
- For patients that have previously received gene therapy, supportive documentation showing response to gene therapy

*Laboratory tests should be obtained within several days prior to administration

COVERAGE CRITERIA

Submission of medical records (chart notes) related to the medical necessity criteria is REQUIRED on all requests for authorizations. Records will be reviewed at the time of submission. Please provide documentation related to diagnosis, step therapy, and clinical markers (i.e. genetic and mutational testing) supporting initiation when applicable. Please provide documentation via direct upload through the PA web portal or by fax.

Spinal Muscular Atrophy (SMA)

Nusinersen (Spinraza) is considered **medically appropriate** if **ALL** of the following conditions are met:

- Patient must not have previously received treatment with SMA gene therapy (i.e., Zolgensma, Itvisma); **OR**
  - Patient previously received treatment with SMA gene therapy (i.e., Zolgensma, Itvisma) and has residual significant functional impairment (*Note: these requests will be reviewed on a case-by-case basis)*
- Patient will not use in combination with other agents for SMA (e.g., Zolgensma, Itvisma, Evrysdi, etc.)
  - NOTE: Use of Spinraza in combination with Isembyld is permitted
- Patient must not have advanced disease (complete limb paralysis, permanent ventilation support, etc.)
- Patient must have the following laboratory tests at baseline and prior to each administration*: platelet count, prothrombin time; activated partial thromboplastin time, and quantitative spot urine protein testing
- Patient retains meaningful voluntary motor function (e.g., manipulate objects using upper extremities, ambulate, etc.)
- Patient must have a diagnosis of 5q spinal muscular atrophy confirmed by either homozygous deletion of the SMN1 gene or dysfunctional mutation of the SMN1 gene
- Patient must have a diagnosis of SMA phenotype I, II or III; **AND**
  - Patient has ≤ 3 copies of the SMN2 gene (*Note: Patients with >3 copies of the SMN2 gene will be reviewed on a case-by-case basis*); **OR**
  - Patient has symptomatic disease (i.e., impaired motor function and/or delayed motor milestones)
- The loading dose regimen is administered in accordance with the FDA-approved prescribing information (see Appendix A)

CONTINUATION OF THERAPY

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for spinal muscular atrophy when all of the following criteria are met:

- Patient continues to meet the criteria such as concomitant therapy requirements (not including prerequisite therapy), performance status, etc. identified in the coverage criteria
- Absence of unacceptable toxicity which would preclude safe administration of the drug. Examples of unacceptable toxicity include: significant renal toxicity, thrombocytopenia, coagulation abnormalities, etc.
- Patient has responded to therapy compared to pretreatment baseline in one or more of the following:
  - Stability or improvement in net motor function/milestones, including but not limited to, the following: validated scales: Hammersmith Infant Neurologic Exam (HINE), Hammersmith Functional Motor Scale Expanded (HFMSE), Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND), Bayley Scales of Infant and Toddler development Third Ed. (BSID-III), 6-minute walk test (6MWT), upper limb module (ULM), motor function measure 32 (MFM32), revised upper limb module (RULM), etc.

- Stability or improvement in respiratory function tests [e.g., (FVC), etc.]
- Reduction in exacerbations necessitating hospitalization and/or antibiotic therapy for respiratory infection in the preceding year timeframe
- Stable or increased patient weight (for patients without a gastrostomy tube)

- The maintenance dose regimen is administered in accordance with the FDA-approved prescribing information (see Appendix A)

APPENDIX

APPENDIX A. Dosing Recommendations

Regimen	Dose (Volume)	Administration Schedule
Low-Dose Regimen	12 mg (5 mL)	Day 0, 14, 28, and 58
High-Dose Regimen	50 mg (5 mL)	Day 0, 14
Low-Dose Regimen (Maintenance)	12 mg (5 mL)	Every 4 months
High-Dose Regimen (Maintenance)	28 mg (5 mL)	Every 4 months

APPLICABLE TENNESSEE STATE MANDATE REQUIREMENTS

BlueCross BlueShield of Tennessee's Medical Policy complies with Tennessee Code Annotated Section 56-7-2352 regarding coverage of off-label indications of Food and Drug Administration (FDA) approved drugs when the off-label use is recognized in one of the statutorily recognized standard reference compendia or in the published peer-reviewed medical literature.

ADDITIONAL INFORMATION

For appropriate chemotherapy regimens, dosage information, contraindications, precautions, warnings, and monitoring information, please refer to one of the standard reference compendia (e.g., the NCCN Clinical Practice Guidelines in Oncology (NCCN Guidelines®) published by the National Comprehensive Cancer Network®, Drugdex Evaluations of Micromedex Solutions at Truven Health, or The American Hospital Formulary Service Drug Information).

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EFFECTIVE DATE

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