RX.PA.035.MPC Spinraza (Nusinesen)

The purpose of this policy is to define the prior authorization process for Spinraza (nusinersen).

DEFINITIONS

Brooke Upper Extremity Functional Scale – measurement of motor function. It scores upper extremity function from 1 (can elevate arms full range to the head), 2 (can elevate arms but needs to flex elbow or use accessory muscles) 3 and 4 (unable to elevate the shoulders but can raise hands to the mouth with or without weight respectively), 5 (unable to raise hands to the mouth and only some hand movement exists), to 6 (no useful
function of hands).

Children's Hospital of Philadelphia Infant Test for Neuromuscular Disorders (CHOP-INTEND) – measurement of motor function in infants. It scores motor function (0: worst to 4: best) via 16 different items, which capture neck, trunk, proximal, and distal limbs.

Hammersmith Infant Neurological Exam, Section 2 (HINE-2) – measurement of functional ability and achievement of motor milestones in infants. It scores seven different areas of motor milestone development, with a maximum score between 2-4 points for each, depending on the milestone, and a total maximum score of 26.

Myometry – measurement of muscle strength with an apparatus.

Spinal Muscular Atrophy (SMA) – an autosomal recessive neuromuscular disease characterized by degeneration of the motor neurons in the anterior horn of the spinal cord, resulting in atrophy of the voluntary muscles of the limbs and trunk. Despite being a rare disease, SMA is a leading genetic cause of infant mortality and a major cause of childhood morbidity. It is attributed to deletions or mutations in the *SMN1* gene (chromosome 5q13), causing insufficient expression of survival motor neuron (SMN) protein. The lack of SMN protein appears to result in dysfunction and eventual death of motor neurons. SMA can present clinically at any time from in utero to adulthood with gross motor function deficits, muscle weakness, and pulmonary disease due to neuromuscular weakness. Common complications include: difficulty feeding, swallowing, failure to thrive, loss of ambulation, scoliosis, joint contracture, pulmonary disease, and death.

Spinal Muscular Atrophy Types I, II, III – Type I manifests around or before the patient is 6 months of age. The presentation may include: hypotonia, unable to control head movement, unable to sit without assistance. Type II has an onset between 6 to 18 months. Patients are generally able to sit independently, the ability to walk is usually not achieved without assistance. Type III manifests after the patient is 18 months of age or older. Patients may be able to walk without assistance or lose the ability to walk.

The drug, Spinraza (nusinersen), is subject to the prior authorization process.

PROCEDURE

A. Initial Authorization Criteria:

Must meet all of the criteria listed below:

- Must be prescribed by a neurologist who specializes in the treatment of spinal muscular atrophy
- Must have a diagnosis of spinal muscular atrophy type I, II, or III. Chart documentation of confirmatory genetic testing demonstrating one of the following in the SMN1 gene is required:
  - 5q SMA Homozygous gene deletion
  - 5q SMA Homozygous gene mutation
  - 5q SMA Compound heterozygote gene mutation
- Must be willing to meet medical care guidelines for the care of the member (e.g., nutritional, respiratory, orthopedic)
- Must not have spinal hardware precluding an intrathecal injection (growing rods are acceptable)
- Must provide chart documentation of baseline motor function and/or strength (e.g., Brooke upper extremity functional score, HINE-2 score, CHOP-INTEND score, myometry measurement)
- Must have chart documentation of baseline and subsequent plan for laboratory monitoring for thrombocytopenia, coagulation abnormalities, and elevated urine protein via all of the following tests:
  - Platelet count
  - Prothrombin time and activated partial thromboplastin time
  - Quantitative spot urine protein testing
- Must not have previously been treated with Zolgensma

B. Must be prescribed at a dose within the manufacturer's dosing guidelines (based on diagnosis, weight, etc) listed in the FDA approved labeling.

C. Spinraza will be considered investigational or experimental for any other use and will not be covered.

D. Reauthorization Criteria:

All prior authorization renewals are reviewed on an annual basis to determine the Medical Necessity for continuation of therapy. Authorization may be extended at 1 year intervals based upon all of the following:

- MPC Renewal:
  - Documentation from the neurologist that the member remains a candidate for treatment with Spinraza (nusinersen) based upon the prescriber's assessment while on therapy
  - Documentation that the member's motor function and/or strength has stabilized as compared to baseline
  - Chart documentation confirming that laboratory tests (within 30 days of request) are performed prior to each dose to monitor for thrombocytopenia, coagulation abnormalities, and elevated urine protein
  - Must not have previously been treated with Zolgensma
- Renewal from Previous Insurer:
  - Members who have received prior approval (from insurer other than MPC), or have been receiving medication samples, should be considered under criterion A (Initial Authorization Criteria).
  - Documentation from the neurologist of a clinical response of the member's motor function and/or strength which has stabilized or improved compared to baseline.
  - Chart documentation confirming that laboratory tests (within 30 days of request) are performed prior to each dose to monitor for thrombocytopenia, coagulation abnormalities, and elevated urine protein

Limitations:

Length of Authorization (if above criteria met)	
Initial Authorization	Up to 6 months
Reauthorization	Up to 1 year

If the established criteria are not met, the request is referred to a Medical Director for review, if required for the plan and level of request.

HCPCS Code(s):

Code	Description
J2326	Injection, nusinersen, 0.1 mg

REFERENCES

1. Spinraza [prescribing information]. Cambridge, MA Biogen, Inc.; 2016.
2. Biogen, Inc. SPINRAZATM (nusinersen) injection Dossier V1, 12/30/2016.
3. Brooke MA, et al. Duchenne muscular dystrophy: patterns of clinical progression and effects of supportive therapy. *Neurology* 1989; 39:475-481.
4. Glanzman AM, et al. The Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND): Test development and reliability. *Neuromuscul Disord 2010;* 20:155–161.
5. Haataja, et al. Optimality score for the neurological examination of the infant at 12 and 18 months of age. *J Pediatr* 1999; 135:153-61.
6. Carre A, et al. Review of spinal muscular atrophy (SMA) for prenatal and pediatric genetic counselors. *J Genet Couns* 2016; 25:32-43.
7. Wang CH, et al. Consensus statement for standard of care in spinal muscular atrophy. *J Child Neurol*.2007; 22(8):1027-49.

REVIEW HISTORY

DESCRIPTION OF REVIEW / REVISION	DATE APPROVED
Annual Review *Change in Non-MPC renewal to renewal from previous insurer*	*02/2024*
Selected Revision *Addition of laboratory requirements within 30 days of request* *Addition of documentation requirement from a neurology specialist*	*10/2023*
Annual review	*02/2023*
Selected Revision *Addition of MPC vs Non-MPC Renewal Criteria*	*08/2022*
Annual review	*02/2022*
Addition of dosing requirements and off-label restrictions	*12/2021*
P&T Review	*11/2020*