RX.PA.035.CCH SPINRAZA® (NUSINERSEN)

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

Spinraza® (nusinersen) is indicated for the treatment of spinal muscular atrophy (SMA) in pediatric and adult patients.

POLICY

It is the policy of the Health Plan to maintain a prior authorization process that promotes appropriate utilization of specific drugs with potential for misuse or limited indications. This process involves a review using Food and Drug Administration (FDA) criteria to make a determination of Medical Necessity, and approval by the Medical Policy Committee.

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

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.

PROCEDURE

Initial Authorization Criteria:

Must meet all the criteria listed below:

- Must be prescribed by a neurologist who specializes in the treatment of spinal muscular atrophy and is experienced in performing lumbar puncture
- Must have a diagnosis of 5q spinal muscular atrophy type I, II, or III with chart documentation of confirmatory genetic testing showing ONE of the following in the SMN1 gene:
    - Homozygous gene deletion (e.g., homozygous deletion of exon 7 at locus 5q13)
    - Homozygous gene mutation
    - Compound heterozygote gene mutation [e.g., deletion of SMN1 exon 7 (allele 1) and mutation of SMN1 (allele 2)]
- Must have chart documentation of genetic testing confirming at least 2 copies of SMN2
- 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 NOT be dependent on either invasive ventilation or tracheostomy, OR non-invasive ventilation for at least 12 hours per day
- Must meet ONE of the following:
    - Member has not previously received gene replacement therapy for treatment of SMA OR
    - Member has previously received gene replacement therapy AND has experienced a decline in clinical status that represents a potential failure or abatement of gene therapy efficacy

- Must have chart documentation of baseline and subsequent plan for pre-dose 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

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 the following:

- Documentation from the provider 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 are performed prior to each dose to monitor for thrombocytopenia, coagulation abnormalities, and elevated urine protein
- Documentation confirming the member does not have respiratory dependency on either:
    - Invasive ventilation or tracheostomy
    - Non-invasive ventilation for a period of 6 or more hours per day
- Member must not be receiving concomitant SMA treatment (i.e., Zolgensma, Evrysdi)

Limitations:

Length of Authorization (if above criteria met)	
Initial Authorization	Up to 1 year
Reauthorization	Same as initial

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)

HCPCS Code	Brand	Description
J2326	SPINRAZA	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
Initial Review	3/22
Updated authorization duration to 1 year	*2/23*

Record Retention

Records Retention for Evolent Health documents, regardless of medium, are provided within the Evolent Health records retention policy and as indicated in CORP.028.E Records Retention Policy and Procedure.

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