Medical Policy

Spinraza™ (nusinersen)	
MEDICAL POLICY NUMBER	MED_Clin_Ops_026
ORIGINAL EFFECTIVE DATE	December 7, 2020
CURRENT VERSION EFFECTIVE DATE	January 1, 2024
APPLICABLE PRODUCT AND MARKET	*Individual Family Plan:* All Plans *Small Group*: All Plans *Medicare Advantage:* All Plans

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PURPOSE

To promote consistency between reviewers in clinical coverage decision-making by

providing the criteria that generally determine the medical necessity of Spinraza™ (nusinersen) therapy.

POLICY/CRITERA

Prior Authorization and Medical Review is required.

Spinraza is considered medically necessary for the treatment of Types I, II, or III SMA in pediatric and adult patients when the following criteria are met.

Initiation therapy; all:

1. Diagnosis of SMA by, or in consultation with a neurologist with expertise of SMA; **AND**
2. Spinraza is being prescribed by, or in consultation with a neurologist with expertise of SMA; **AND**
3. Clinical documentation of 5q SMA homozygous gene mutation, homozygous gene deletion, or compound heterozygote (a or b, AND c)
   a. Homozygous gene deletion or mutation (e.g., homozygous deletion of exon 7 at locus 5q13); **OR**
   b. Compound heterozygous mutation (e.g., deletion of SMN1 exon 7 [allele 1] and mutation of SMN1 [allele 2]); **AND**
   c. Patient has Documentation of genetic testing confirming at least 2 copies of SMN2; **AND**
4. Baseline exam of at least ONE of the following exams to establish baseline motor ability:
   a. Hammersmith Infant Neurological Exam (HINE) (infant to early childhood); **OR**
   b. Hammersmith Functional Motor Scale Expanded (HFMSE); **OR**
   c. Upper Limb Module (ULM) Test (Non ambulatory); **OR**
   d. Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND); **AND**
5. Patient is NOT dependent on either invasive ventilation or tracheostomy, OR Non-invasive ventilation for at least 12 hours per day; **AND**
6. Patient meets one of the following (a or b):
   a. Patient has not previously received gene replacement therapy for the treatment of SMA; **OR**
   b. Patient meets both of the following (i and ii):
      i. Patient has previously received gene replacement therapy; **AND**
      ii. Patient has experienced a decline in clinical status that represented a potential failure or abatement of gene therapy efficacy; **AND**
7. Spinraza is to be administered intrathecally by, or under the direction of, healthcare professionals experienced in performing lumbar punctures; **AND**
8. Spinraza dosing is in accordance with the United States Food and Drug Administration approved labeling: maximum dosing of 12mg for each loading dose; **AND**
9. Initial authorization will be for no more than 4 loading doses; **AND**

Continuation therapy; all (1-5):

1. Diagnosis of SMA by, or in consultation with, a neurologist with expertise of SMA; **AND**
2. Patient has previously been treated with Spinraza; **AND**
3. Patient does not have respiratory dependency on either:
    a. Invasive ventilation or tracheostomy; **OR**
    b. Non-invasive ventilation for a period ≥ 6 hours per day; **AND**
3. Patient has not been previously treated with gene replacement therapy for the treatment of SMA; **AND**
4. Patient is not receiving concomitant SMA treatment (i.e., Zolgensma [onasemnogene abeparvovec-xioi], Evrysdi [risdiplam]); **AND**
5. Clinical documentation demonstrating a positive therapeutic response to Spinraza, from pretreatment baseline, as documented by at least one of the following (a, b, c, or d, as appropriate) (provider evaluation must occur ≤ 1 month prior to request):
    a. Hammersmith Infant Neurological Examination (HINE) milestones (for infants 2 months–2 years of age (i and ii):
        i. One of the following:
            1. Improvement, or maintenance of previous improvement, of at least 2-point (or maximal score) increase in ability to kick; **OR**
            2. Improvement, or maintenance of previous improvement, of at least 1-point increase in any other HINE milestone (e.g., head control, rolling, sitting, crawling, etc.), excluding voluntary grasp; **OR**
        ii. One of the following:
            1. Improvement or maintenance of previous improvement in more HINE motor milestones than worsening, from pretreatment baseline (net positive improvement) ; **OR**
            2. Patient achieved and maintained any new motor milestones that is otherwise not expected (e.g., sit unassisted, stand, walk) ; **OR**
    b. Hammersmith Functional Motor Scale (HFMSE): (i. or ii.)
        i. Improvement, or maintenance of previous improvement, of at least a 3-point increase in score from pretreatment baseline; **OR**
        ii. Patient has achieved and maintained any new motor milestone from pretreatment baseline that is otherwise not expected; **OR**
    c. Upper Limb Module (ULM): (i. or ii.)
        i. Improvement or maintenance of previous improvement of at least a 2-point increase in score from pretreatment baseline; **OR**
        ii. Patient has achieved and maintained any new motor milestone from pretreatment baseline that is otherwise not expected; **OR**
    d. Children's Hospital of Philadelphia (CHOP) infant Test of Neuromuscular Disorders (INTEND): (i. or ii.)
        i. Improvement, or maintenance of previous improvement, of at least a 4-point increase in score from pretreatment baseline
        ii. Patient has achieved and maintained any new motor milestone from pretreatment baseline that is otherwise not expected; **AND**

6. Spinraza is to be administered intrathecally by, or under the direction of, healthcare professionals experienced in performing lumbar punctures; **AND**
7. Total dose not to exceed 1 vial (12 mg) every 4 months; **AND**
8. Duration of continuation of therapy approval is 12 months (3 maintenance doses)

EXCLUSIONS

1. Spinraza is not considered medically necessary for any indication other than as listed above.
2. Spinraza is not considered medical necessary for individuals in current treatment or previously treated with gene therapy for SMA

DEFINITIONS

Spinraza is a survival motor neuron-2 (SMN2)-directed antisense oligonucleotide indicated for the treatment of spinal muscular atrophy (SMA). The drug is administered intrathecally.

Spinal muscular atrophy (SMA) is neurological disease characterized by loss of motor neurons in the spinal cord and lower brain stem, resulting in severe and progressive muscular atrophy and weakness. 5q-SMA is an autosomal recessive genetic disorder caused by mutations in the SMN1 (survival motor neuron) gene that is found on chromosome 5. To develop SMA, an individual must inherit two faulty SMN1 genes, one from each parent.

- **SMA Type 1** (infantile onset SMA or Werdnig-Hoffmann disease) — symptoms are present at birth or by the age of 6 months
- **SMA Type 2** — onset of symptoms between the ages of 7 and 18 months and before the child can stand or walk independently
- **SMA Type 3** — onset of symptoms after 18 months, and children can stand and walk independently, although they may require aids
- **SMA Type 4** (adult-onset SMA or Kugelberg-Welander disease) — onset of symptoms in adulthood, and people are able to walk during their adult years.

CODING

Applicable Procedure Codes

J2326	Injection, nusinersen, 0.1 mg (Eff. 1/1/2018)
J3490	Unclassified drugs (prior to 1/1/2018)
J3590	Unclassified biologics (prior to 1/1/2019)

Applicable Diagnosis Codes

G12.0	Infantile spinal muscular atrophy, type I [Werdnig-Hoffman]
G12.1	Other inherited spinal muscular atrophy
G12.8	Other spinal muscular atrophies and related syndromes
G12.9	Spinal muscular atrophy, unspecified

Applicable NDCS

64406-0058-01	Spinraza 5ml Vial

EVIDENCE BASED REFERENCES

Spinraza

1. Med Lett Drugs Ther. 2017 Mar 27;59(1517):50-52. Nusinersen (Spinraza) for spinal muscular atrophy.
2. Nat Neurosci. 2017 Apr;20(4):497-499. doi: 10.1038/nn.4508. Epub 2017 Feb 13. Nusinersen, an antisense oligonucleotide drug for spinal muscular atrophy. Corey DR.
3. Neurology. 2016 Mar 8;86(10):890-7. doi: 10.1212/WNL.0000000000002445. Epub 2016 Feb 10. Results from a phase 1 study of nusinersen (ISIS-SMN(Rx)) in children with spinal muscular atrophy. Chiriboga CA1, Swoboda KJ2, Darras BT2, Iannaccone ST2, Montes J2, De Vivo DC2, Norris DA2, Bennett CF2, Bishop KM2.
4. Specialty matched clinical peer review.
5. Spinraza [Prescribing Information] Cambridge, MA: Biogen; December 2016

POLICY HISTORY

Original Effective Date	December 7, 2020
Revised Date	November 1, 2021 – Annual review and approval (no policy revisions made) January 1, 2024 - Updated to Brand New Day/Central Health Medicare Plan (no policy revisions made)

Approved by Pharmacy and Therapeutics 11/1/2021