Policy Title:	Spinraza (nusinersen) (intrathecal)		
		Department:	PHA
Effective Date:	04/10/2019		
Review Date:	04/10/2019, 7/26/2019, 1/15/2020, 8/24/2020, 01/12/2021, 01/20/2022, 2/23/2023, 12/07/2023, 01/04/2024, 03/12/2025		

Purpose: To support safe, effective, and appropriate use of Spinraza (nusinersen) in the treatment of spinal muscular atrophy in pediatric and adult patients.

Scope: Medicaid, Commercial, Medicare-Medicaid Plan (MMP)

Policy Statement: Spinraza (nusinersen) is covered under the Medical Benefit when used within the following guidelines. Use outside of these guidelines may result in non-payment unless approved under an exception process.

Procedure: Coverage of Spinraza (nusinersen) will be reviewed prospectively via the prior authorization process based on criteria below:

Summary of Evidence: Spinraza is a survival motor neuron-2 (SMN2)-directed antisense oligonucleotide indicated for the treatment of spinal muscular atrophy (SMA) in pediatric and adult patients. Clinical trials evaluating the efficacy and safety of Spinraza in patients with SMA have demonstrated significant therapeutic benefits, including improved motor function, increased survival, and delayed disease progression. Common adverse events are mild to moderate injection site reactions and respiratory infections.

Initial Criteria Coverage:

- Patient must have the following laboratory tests at baseline and prior to each administration (laboratory tests should be obtained within several days prior to administration): platelet count, prothrombin time, activated partial thromboplastin time, and quantitative spot urine protein testing; AND
- Patient retains voluntary motor function (e.g. manipulate objects using upper extremities, ambulate, etc.); AND
- 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; AND
- Patient has at least 2 copies of SMN2; AND
- Patient has not received a dose of Zolgensma (onasemnogene abeparvovec-xioi) in the past and will not be used concurrently with Spinraza (nusinersen); AND
- Patient will not be using Spinraza (nusinersen) in combination with Evrysdi (risdiplam); AND
- Patient is not dependent on either of the following:
    - Invasive ventilation or tracheostomy.

- Use of non-invasive ventilation beyond the use for naps and nighttime sleep; AND

- Patient must have a diagnosis of SMA phenotype I, II, or III; AND
  - Patient has ≤ 3 copies of the *SMN2* gene; OR
  - Patient has symptomatic disease (i.e., impaired motor function and/or delayed motor milestones); AND
- 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), 6-minute walk test (6MWT), upper limb module (ULM), 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)
- MMP members who have previously received this medication within the past 365 days are not subject to Step Therapy Requirements

Continuation of therapy:

- Patient meets all initial criteria; AND
- Patient is tolerating treatment; AND
- Patient has not received a dose of Zolgensma (onasemnogene abeparvovec-xioi) in the past and will not be used concurrently with Spinraza (nusinersen) or being used in combination with Evrysdi (risdiplam); AND
- Recent laboratory values (i.e. platelet count, prothrombin time, activated partial thromboplastin time, and quantitative spot urine protein testing) must be submitted associated with last dose given; AND
- Patient has responded to therapy compared to pretreatment baseline (e.g., chart notes) by two or more of the following:

  - Prescriber must submit medical records (e.g., chart notes, laboratory values) with the most recent results documenting a positive clinical response from pretreatment baseline status to Spinraza therapy as demonstrated by at least one of the following exams:

    A. HINE-2 milestones:
       One of the following:
    - Improvement or maintenance of previous improvement of at least 2 point (or maximal score) increase in ability to kick.
    - Improvement or maintenance of previous improvement of at least 1 point increase in any other HINE-2 milestone (e.g., head control, rolling, sitting, crawling, etc.) excluding voluntary grasp; AND

      One of the following:

- The patient exhibited improvement or maintenance of previous improvement in more HINE motor milestones than worsening, from pretreatment baseline (net positive improvement).
- Achieved and maintained any new motor milestones when they would otherwise be unexpected to do so (e.g., sit unassisted, stand, walk); OR

B. HFMSE:

One of the following:

- Improvement or maintenance of previous improvement of at least a 3 point increase in score from pretreatment baseline.
- Patient has achieved and maintained any new motor milestone from pretreatment baseline when they would otherwise be unexpected to do so; OR

C. ULM:

One of the following:

- Improvement or maintenance of previous improvement of at least a 2 point increase in score from pretreatment baseline.
- Patient has achieved and maintained any new motor milestone from pretreatment baseline when they would otherwise be unexpected to do so; OR

D. CHOP INTEND:

One of the following:

- Improvement or maintenance of previous improvement of at least a 4 point increase in score from pretreatment baseline.
- Patient has achieved and maintained any new motor milestone from pretreatment baseline when they would otherwise be unexpected to do so;

- Stability or improvement in respiratory function tests (such as forced vital capacity [FVC], etc.)
- Reductions in exacerbations necessitating hospitalization and/or antibiotic therapy for respiratory infection in the preceding year/timeframe
- Stable or increased weight (for patient's without a gastrostomy tube)
- Slowed rate of decline in the aforementioned measures

Coverage durations:

- Initial coverage criteria = 6 months
- Continuation of therapy = 12 months

Dosing:

- Initial dose: 120 billable units on day 0, day 14, day 28, day 59 (480 units)
- Renewal: 120 billable units every 120 days (360 units)

Per §§ 42 CFR 422.101, this clinical medical policy only applies to INTEGRITY in the absence of National Coverage Determination (NCD) or Local Coverage Determination (LCD).

Policy Rationale:
Spinraza was reviewed by the Neighborhood Health Plan of Rhode Island Pharmacy & Therapeutics (P&T) Committee. Neighborhood adopted the following clinical coverage criteria to ensure that its members use Spinraza according to Food and Drug Administration (FDA) approved labeling and/or relevant clinical literature. Neighborhood worked with network prescribers and pharmacists to draft these criteria. These criteria will help ensure its members are using this drug for a medically accepted indication, while minimizing the risk for adverse effects and ensuring more cost-effective options are used first, if applicable and appropriate. For INTEGRITY (Medicare-Medicaid Plan) members, these coverage criteria will only apply in the absence of National Coverage Determination (NCD) or Local Coverage Determination (LCD) criteria. Neighborhood will give individual consideration to each request it reviews based on the information submitted by the prescriber and other information available to the plan.

Investigational Use: All therapies are considered investigational when used at a dose or for a condition other than those that are recognized as medically accepted indications as defined in any one of the following standard reference compendia: American Hospital Formulary Service Drug Information (AHFS-DI), Thomson Micromedex DrugDex, Clinical Pharmacology, Wolters Kluwer Lexi-Drugs, or Peer-reviewed published medical literature indicating that sufficient evidence exists to support use. Neighborhood does not provide coverage for drugs when used for investigational purposes.

Applicable Codes: Below is a list of billing codes applicable for covered treatment options. The below tables are provided for reference purposes and may not be all-inclusive. Requests received with codes from tables below do not guarantee coverage. Requests must meet all criteria provided in the procedure section.

The following HCPCS/CPT codes are:

HCPCS/CPT Code	Description
J2326	Injection, nusinersen, 0.1mg

References:

1. Spinraza prescribing information. Cambridge, MA.: Biogen, Inc.; April 2024. accessed February 2025.
2. Markowitz JA, Singh P, Darras BT. Spinal Muscular Atrophy: A Clinical and Research Update. Pediatric Neurology 46 (2012) 1-12.
3. Sugarman EA, Nagan N, Zhu H, et al. Pan-ethnic carrier screening and prenatal diagnosis for spinal muscular atrophy: clinical laboratory analysis of >72,400 specimens. Eur J Hum Genet 2012;20:27-32.
4. Prior TW, Snyder PJ, Rink BD, et al. Newborn and carrier screening for spinal muscular atrophy. Am J Med Genet A. 2010 Jul;152A(7):1608-16.