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		

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:

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)

*** Requests will also be reviewed to National Coverage Determination (NCD) and Local Coverage Determinations (LCDs) if applicable.***

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.; December 2022 .
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.