Policy Title:	Spinraza (nusinersen) (intrathecally)		
Policy Number:	000694	**Department:**	PHA
Effective Date:	04/10/2019		
Review Date:	04/10/2019, 7/26/2019		
Revision Date:	04/10/2019, 7/26/2019		

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, Exchange, Integrity

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 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 one of the following SMA phenotypes:
    - SMA I
    - SMA II with symptomatic disease (i.e. impaired motor function and/or delayed motor milestones).
    - SMA III with 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), 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).

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); 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 one or more of the following:

1. 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:

a. Improvement or maintenance of previous improvement of at least 2 point (or maximal score) increase in ability to kick.
b. 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:

a. The patient exhibited improvement or maintenance of previous improvement in more HINE motor milestones than worsening, from pretreatment baseline (net positive improvement).
b. 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:

a. Improvement or maintenance of previous improvement of at least a 3 point increase in score from pretreatment baseline.
b. 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:

a. Improvement or maintenance of previous improvement of at least a 2 point

increase in score from pretreatment baseline.

b. 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:

a. Improvement or maintenance of previous improvement of at least a 4 point increase in score from pretreatment baseline.

b. Patient has achieved and maintained any new motor milestone from pretreatment baseline when they would otherwise be unexpected to do so;

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

Coverage durations:

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

Dosing:

- Initial dose: 120 billable units on day 0, day 14, day 28, day 58, and day 178
- Renewal: 120 billable units every 120 days

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.

Additional Information:

Indications:

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

Dosing:

- The first three loading doses (12mg) should be administered at 14-day intervals. The 4th loading dose (12mg) should be administered 30 days after the 3rd dose. A maintenance dose (12mg) should be administered once every 4 months thereafter.

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.; 2018 October.
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.