Spinraza® (nusinersen)
(Intrathecal)

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I. Length of Authorization

Coverage will be provided annually and may be renewed.

II. Dosing Limits

A. Quantity Limit (max daily dose) [NDC Unit]:

- Loading: 1 vial on D1, D15, D29, and D59
- Maintenance: 1 vial every 112 days

B. Max Units (per dose and over time) [HCPCS Unit]:

- Loading: 120 billable units on D1, D15, D29, and D59
- Maintenance: 120 billable units every 112 days

III. Initial Approval Criteria[1-12]

Submission of medical records (chart notes) related to the medical necessity criteria is REQUIRED on all requests for authorizations. Records will be reviewed at the time of submission. Please provide documentation related to diagnosis, step therapy, and clinical markers (i.e. genetic and mutational testing) supporting initiation when applicable. Please provide documentation via direct upload through the PA web portal or by fax.

Coverage is provided in the following conditions:

Spinal Muscular Atrophy (SMA) † Φ

- Patient must not have previously received treatment with SMA gene therapy (i.e., onasemnogene abeparvovec-xioi); **AND**
- Patient will not use in combination with other agents for SMA (e.g., onasemnogene abeparvovec, risdiplam, etc.); **AND**
- Patient must not have advanced disease (complete limb paralysis, permanent ventilation support, etc.); **AND**

- Patient must have the following laboratory tests at baseline and prior to each administration*: platelet count, prothrombin time; activated partial thromboplastin time, and quantitative spot urine protein testing; **AND**
- Patient retains meaningful 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 must have a diagnosis of SMA phenotype I, II, or III; **AND**
    - Patient has ≤ 3 copies of the *SMN2* gene *(Note: Patients with >3 copies of the SMN2 gene will be reviewed on a case-by-case basis)*; **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), Bayley Scales of Infant and Toddler development Third Ed. (BSID-III), 6-minute walk test (6MWT), upper limb module (ULM), motor function measure 32 (MFM32), revised upper limb module (RULM), 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)

*Laboratory tests should be obtained within several days prior to administration

† FDA-labeled indication(s), ‡ Compendia recommended indication(s); Φ Orphan Drug

IV. Renewal Criteria[1]

- Patient continues to meet universal and other indication-specific relevant criteria such as concomitant therapy requirements (not including prerequisite therapy), performance status, etc. identified in section III; **AND**
- Absence of unacceptable toxicity which would preclude safe administration of the drug. Examples of unacceptable toxicity include the following: significant renal toxicity, thrombocytopenia, coagulation abnormalities, etc.; **AND**
- Patient has responded to therapy compared to pretreatment baseline in one or more of the following:

- Stability or improvement in net 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), Bayley Scales of Infant and Toddler development Third Ed. (BSID-III), 6-minute walk test (6MWT), upper limb module (ULM), motor function measure 32 (MFM32), revised upper limb module (RULM), etc.
- Stability or improvement in respiratory function tests [e.g., forced vital capacity (FVC), etc.]
- Reduction in exacerbations necessitating hospitalization and/or antibiotic therapy for respiratory infection in the preceding year/timeframe
- Stable or increased patient weight (for patients without a gastrostomy tube)
- Slowed rate of decline in the aforementioned measures

V. Dosage/Administration

Indication	Dose
Spinal Muscular Atrophy	12 mg administered, as an intrathecal bolus injection over 1 to 3 minutes using a spinal anesthesia needle, per administration. Prior to administration, 5 mL of cerebrospinal fluid should be removed. Imaging guidance and sedation may be required for administration. Initiation Four loading doses: the first three loading doses should be administered at 14-day intervals. The 4th loading dose should be administered 30 days after the 3rd dose. Maintenance One dose every 4 months (112 days) thereafter

Store refrigerated at 2ºC to 8ºC; warm to room temperature prior to administration

VI. Billing Code/Availability Information

HCPCS code:

- J2326 –Injection, nusinersen, 0.1 mg; 1 billable unit = 0.1 mg

NDC:

- Spinraza 12 mg/5 mL solution for injection; single-dose vial: 64406-0058-xx

VII. References

1. Spinraza [package insert]. Cambridge, MA; Biogen, Inc.; June 2020. Accessed July 2022.
2. Wang CH, Finkel RS, Bertini ES, et al. Consensus statement for standard of care in spinal muscular atrophy. J Child Neurol. 2007 Aug;22(8):1027-49.
3. Prior TW, Finanger E. Spinal muscular atrophy. GeneReviews. www.ncbi.nlm.nih.gov/books/NBK1352/ (Accessed on June 10, 2019).
4. Finkel RS, Mercuri E, Darras BT, et al; for the ENDEAR Study Group. Nusinersen versus sham control in infantile-onset spinal muscular atrophy. N Engl J Med. 2017;377(18):1723-

1732.Finkel RS, Mercuri E, Darras BT, et al; for the ENDEAR Study Group. Nusinersen versus sham control in infantile-onset spinal muscular atrophy. N Engl J Med. 2017;377(18):1723-1732.
5. Mercuri E, Darras BT, Chiriboga CA, et al; for the CHERISH Study Group. Nusinersen versus sham control in later-onset spinal muscular atrophy. N Engl J Med. 2018;375:625-635.
6. Dabbous O, Maru B, Jansen JP, et al. Survival, Motor Function, and Motor Milestones: Comparison of AVXS-101 Relative to Nusinersen for the Treatment of Infants with Spinal Muscular Atrophy Type 1. Adv Ther. 2019 May;36(5):1164-1176.
7. Kichula E, Duong T, Glanzman A, et al. Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND) Feasibility for Individuals with Severe Spinal Muscular Atrophy II (S46.004). Neurology Apr 2018, 90 (15 Supplement) S46.004
8. De Vivo DC, Bertini E, Swoboda KJ, et al. Nusinersen initiated in infants during the presymptomatic stage of spinal muscular atrophy: Interim efficacy and safety results from the Phase 2 NURTURE study. Neuromuscul Disord. 2019 Nov;29(11):842-856. doi: 10.1016/j.nmd.2019.09.007. Epub 2019 Sep 12.
9. Michelson D, Ciafaloni E, Ashwal S, et al. Evidence in focus: Nusinersen use in spinal muscular atrophy: Report of the Guideline Development, Dissemination, and Implementation Subcommittee of the American Academy of Neurology. Neurology. 2018 Nov 13;91(20):923-933. doi: 10.1212/WNL.0000000000006502. Epub 2018 Oct 12.
10. Darras BT, Chiriboga CA, Iannaccone ST, et al. Nusinersen in later-onset spinal muscular atrophy: long-term results from the phase 1/2 studies. Neurology. 2019;92(21):e2492-e2506
11. Finikel RS, Mercuri E, Meyer OH, et al. Diagnosis and management of spinal muscular atrophy: Part 2: Pulmonary and acute care; medications, supplements and immunizations; other organ systems; and ethics. Neuromuscul Disord. 2018 Mar;28(3):197-207. doi: 10.1016/j.nmd.2017.11.004. Epub 2017 Nov 23.
12. (ICER) IfCaER . Spinraza and Zolgensma for Spinal Muscular Atrophy: Effectiveness and Value. Final Evidence Report. April 3, 2019 (Updated May 24, 2019) 2019.
13. Kichula E, Duong T, Glanzman A, et al. Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND) Feasibility for Individuals with Severe Spinal Muscular Atrophy II (S46.004). Neurology Apr 2018, 90 (15 Supplement) S46.004

Appendix 1 – Covered Diagnosis Codes

ICD-10	ICD-10 Description
G12.0	Infantile spinal muscular atrophy, type I [Werdnig-Hoffmann]
G12.1	Other inherited spinal muscular atrophy
G12.25	Progressive spinal muscle atrophy
G12.8	Other spinal muscular atrophies and related syndromes
G12.9	Spinal muscular atrophy, unspecified

Appendix 2 – Centers for Medicare and Medicaid Services (CMS)

Medicare coverage for outpatient (Part B) drugs is outlined in the Medicare Benefit Policy Manual (Pub. 100-2), Chapter 15, §50 Drugs and Biologicals. In addition, National Coverage Determination (NCD) and Local Coverage Determinations (LCDs) may exist and compliance with these policies is required where applicable. They can be found at: http://www.cms.gov/medicare-coverage-database/search.aspx. Additional indications may be covered at the discretion of the health plan.

Medicare Part B Covered Diagnosis Codes (applicable to existing NCD/LCD/LCA): N/A

Medicare Part B Administrative Contractor (MAC) Jurisdictions		
Jurisdiction	**Applicable State/US Territory**	**Contractor**
E (1)	CA, HI, NV, AS, GU, CNMI	Noridian Healthcare Solutions, LLC
F (2 & 3)	AK, WA, OR, ID, ND, SD, MT, WY, UT, AZ	Noridian Healthcare Solutions, LLC
5	KS, NE, IA, MO	Wisconsin Physicians Service Insurance Corp (WPS)
6	MN, WI, IL	National Government Services, Inc. (NGS)
H (4 & 7)	LA, AR, MS, TX, OK, CO, NM	Novitas Solutions, Inc.
8	MI, IN	Wisconsin Physicians Service Insurance Corp (WPS)
N (9)	FL, PR, VI	First Coast Service Options, Inc.
J (10)	TN, GA, AL	Palmetto Government Benefit Administrators, LLC
M (11)	NC, SC, WV, VA (excluding below)	Palmetto GBA, LLC
L (12)	DE, MD, PA, NJ, DC (includes Arlington & Fairfax counties and the city of Alexandria in VA)	Novitas Solutions, Inc.
K (13 & 14)	NY, CT, MA, RI, VT, ME, NH	National Government Services, Inc. (NGS)
15	KY, OH	CGS Administrators, LLC