Washington State Health Care Authority

Nusinersen (Spinraza®)

Medical policy no. 74.70.00-2 Effective Date: August 1, 2018

Background:

Spinal muscular atrophy (SMA) is a rare, hereditary disease characterized by loss of motor neurons in the spinal cord and lower brain stem, and results in severe and progressive muscular atrophy, hypotonia, diffuse symmetric weakness, and restrictive lung disease. Patients with the most severe type of SMA can become paralyzed, never sit or walk, and have difficulty breathing and swallowing due to bulbar muscle weakness (requiring mechanical ventilation, gastrostomy tube enteral feeding, and nursing care).

Medical necessity

Drug	Medical Necessity
nusinersen (Spinraza®)	Nusinersen (Spinraza®) will be considered medically necessary for the treatment of spinal muscular atrophy (SMA) in patients that meet **ALL** the following criteria for initial approval or continuation and none of the exclusion criteria. Documentation from the patient's chart is **REQUIRED**.

Clinical policy:

Clinical Criteria	
Initial Approval Criteria	Nusinersen (Spinraza®) may be covered when **ALL** of the following are met: 1. Patient must have documentation of a confirmed diagnosis of spinal muscular atrophy (SMA) defined as **ONE** of the following (either 1a, 1b, or 1c) genetic tests of 5q13 demonstrating: a. Homozygous SMN1 gene deletion b. Homozygous SMN1 gene mutation c. Compound heterozygous SMN1 gene mutation 2. Patient has sufficient number of copies of SMN2 gene defined as **ONE** of the following (either 2a or 2b) genetic tests demonstrating: a. If a pre-symptomatic infant, then ≤ 3 copies of SMN2 gene is required b. If a symptomatic patient, then ≥ 2 copies of SMN2 gene is required AND documentation of age of onset of symptoms 3. Nusinersen is prescribed by a provider with expertise in treating and managing SMA 4. Nusinersen will be administered by a specialist with competency in intrathecal injections or under the supervision of a provider with expertise in performing lumbar puncture procedures 5. Documentation of at least **TWO** of the following baseline motor exams appropriate for patient age and motor function has been submitted:

	a. Six-Minute Walk Test (6MWT) b. Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND) c. Hammersmith Infant Neurological Exam (HINE) – infant and early childhood d. Hammersmith Functional Motor Scale Expanded (HFMSE) e. Manual Muscle Test (MMT) f. Pulmonary Function Test (PFT) g. Range Of Motion (ROM) h. Revised Upper Limb Module (RULM) Test (non-ambulatory) Upon review of the submitted documentation from the patient's chart and demonstration of meeting the above initial approval criteria, nusinersen will be approved for 5 doses to be administered in a 6 month period. The first 3 doses must be administered 14 days apart, the fourth dose must be 30 days after the third dose, and the fifth dose must be four months after the fourth dose. Continued approval will be required every 8 months for doses to be administered every 4 months.
Continuation Approval Criteria	Continued use Nusinersen (Spinraza®) may be authorized when **ALL** of the following are met: 1. Patient meets the initial approval criteria (items 1-4 above) 2. The documentation of **ONE** applicable patient outcomes, measured within 60 days of request for renewal, has been submitted: See the required assessments above 3. Documentation of **ONE** of the following has been submitted demonstrating medical necessity: a. Improvement in functional status from baseline functional tests (HFMSE, CHOP-INTEND, Pulmonary status, HINE, 6MWT, or RULM). Improvement is defined as the following: i. HFMSE: 1) At least 3 points increase in score from pretreatment baseline ii. CHOP-INTEND: 1) At least a 4 point increase in score from the pretreatment baseline iii. If ambulatory: 6MWT: 1) At least a 30 meter increase from pretreatment baseline iv. If non-ambulatory: RULM: 1) At least a 2 point increase in score from the pretreatment baseline b. Patient has maintained existing motor milestones from pretreatment baseline functional tests (HFMSE, CHOP-INTEND, Pulmonary status, or HINE) c. Disease progression is slower than what would otherwise be expected

	Upon review of the submitted documentation from the patient's chart and demonstration of meeting the above continuation criteria, nusinersen will be approved for an additional 8-month period. Continued approval will be required every 8 months for doses to be administered every 4 months.
Exclusion Criteria	Nusinersen is considered not medically necessary for the treatment of SMA without 5q mutations or deletions or in pre-symptomatic patients with greater than (>) three (3) copies of the SMN2 gene.

Dosage and quantity limits

Dose and Quantity Limits	
Maximum dose	12mg (5mL) per 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

Coding:

Billing Code	Description
J2326	Injection, nusinersen, 0.1 mg
C9489	Injection, nusinersen, 0.1 mg

Definitions

Acronym	Description
6MWT	Six-Minute Walk Test
CHOP INTEND	Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders
HINE	Hammersmith Infant Neurological Exam (infant to early childhood)
HFMSE	Hammersmith Functional Motor Scale Expanded
MMT	Manual Muscle Test
PFT	Pulmonary Function Test
ROM	Range Of Motion
RULM	Revised Upper Limb Module

References

1. Spinraza™ (nusinersen) injection for intrathecal use [package insert]. Cambridge, MA: Biogen, Inc; December 2016

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3. Bodamer, OA. Spinal muscular atrophy (SMA). Last updated Dec. 13, 2016. . In: Nordli DR, Firth, H.V., Martin, R. UpToDate, Waltham, MA, 2016.
4. Product dossier: Spinraza™ (nusinersen) – April 13, 2017. Cambridge, MA: Biogen; Data reviewed May 2017.
5. FDA Center for Drug Evaluation and Research (CDER). Medical Review. NDA 209531; Spinraza (nusinersen). 12/23/2016. [cited 1/25/2017]; Available from: http://www.accessdata.fda.gov/drugsatfda_docs/nda/2016/209531Orig1s000TOC.cfm
6. Wang, CH, Finkel, RS, Bertini, ES, et al. Consensus statement for standard of care in spinal muscular atrophy. Journal of child neurology. 2007 Aug;22(8):1027-49. PMID: 17761659
7. Medical information [data on file]. May 5, 2017. Cambridge, MA: Biogen; Data reviewed May 2017