Washington State Health Care Authority

# Spinal Muscular Atrophy Agents – nusinersen (Spinraza)

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

***Note:*** *New-to-market drugs included in this class based on the Apple Health Preferred Drug List are non-preferred and subject to this prior authorization (PA) criteria. Non-preferred agents in this class require an inadequate response or documented intolerance due to severe adverse reaction or contraindication to at least TWO preferred agents. If there is only one preferred agent in the class documentation of inadequate response to ONE preferred agent is needed. If a drug within this policy receives a new indication approved by the Food and Drug Administration (FDA), medical necessity for the new indication will be determined on a case-by-case basis following FDA labeling.*

*To see the list of the current Apple Health Preferred Drug List (AHPDL), please visit: https://www.hca.wa.gov/assets/billers-and-providers/apple-health-preferred-drug-list.xlsx*

**Related medical policies:**

- **74.70.65 - Spinal Muscular Atrophy Agents – Evrysdi (risdiplam)**

## 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:<br>• Spinal muscular atrophy (SMA) |

## Clinical policy:

| Clinical Criteria | |
|---|---|
| **Initial Approval Criteria** | Nusinersen (Spinraza®) may be covered when **ALL** of the following are met:<br><br>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:<br>a. Homozygous SMN1 gene deletion; **OR**<br>b. Homozygous SMN1 gene mutation; **OR**<br>c. Compound heterozygous SMN1 gene mutation; **AND** |

| | |
|---|---|
| | 2. Patient is symptomatic with a phenotype of SMA I, SMA II, or SMA III; **AND**<br>3. Not used simultaneously with Evrysdi (risdiplam); **AND**<br>4. Patient has not been previously treated with Zolgensma (onasemnogene abeparvovec-xioi); **AND**<br>5. Documentation of at least **ONE** of the following baseline motor exams appropriate for patient age and motor function within the last 90 days:<br>a. Six-Minute Walk Test (6MWT); **OR**<br>b. Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND); **OR**<br>c. Hammersmith Infant Neurological Exam (HINE) – infant and early childhood; **OR**<br>d. Hammersmith Functional Motor Scale Expanded (HFMSE); **OR**<br>e. Revised Upper Limb Module (RULM) test (non-ambulatory); **AND**<br>6. Baseline documentation of **ALL** of the following:<br>a. Neurologic examination; **AND**<br>b. Manual Muscle Test (MMT); **AND**<br>c. Pulmonary Function Test (PFT), if able<br>7. Patient does not require tracheostomy or invasive ventilation; **AND**<br>8. Prescribed by a provider with expertise in treating and managing SMA<br><br>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.<br><br>If all criteria are not met, but there are documented medically necessary or situational circumstances, based on the professional judgement of the clinical reviewer, requests may be approved on a case-by-case basis up to the initial authorization duration. |
| **Continuation Approval Criteria** | Continued use Nusinersen (Spinraza®) may be authorized when **ALL** of the following are met:<br><br>1. Documentation of **ONE** of the following:<br>a. Disease improvement or stability as demonstrated by at least one of the functional scales or motor milestones listed above evaluated in the previous 90 days; **OR**<br>b. Disease progression is slower than what would otherwise be expected<br><br>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.<br><br>If all criteria are not met, but there are documented medically necessary or situational circumstances, based on the professional judgement of the clinical reviewer, requests may be approved on a case-by-case basis up to the reauthorization duration. |
| **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:<br>• The first three loading doses should be administered at 14-day intervals.<br>• The 4th loading dose should be administered 30 days after the 3rd dose. |
| **Maintenance** | One dose every 4 months |

## Definitions:

| Definition | |
|---|---|
| Improvement | • **HFMSE*:** At least 3 points increase in score from pretreatment baseline<br>• **HINE***: More motor milestones have improved than have worsened. Improvement is defined as a 2-point increase in ability to kick OR at least 1 point ability increase in motor milestones of head control, rolling, sitting, crawling, standing or walking.<br>• **CHOP-INTEND*:** At least a 4-point increase in score from the pretreatment baseline<br>• **6MWT (ambulatory):** At least a 30-meter increase from pretreatment baseline<br>• **RULM (non-ambulatory):** At least a 2-point increase in score from the pretreatment baseline |
| Stability | • The functional scale score did not worsen from baseline |

*Improvement is based on minimal clinically important difference in Sprinraza clinical trials

## Coding:

| Billing Code | Description |
|---|---|
| J2326 | Injection, nusinersen, 0.1 mg |

## References


1. Spinraza™ (nusinersen) injection for intrathecal use [package insert]. Cambridge, MA: Biogen, Inc; June 2020
2. Prior TW, Finanger E. Spinal Muscular Atrophy. 2000 Feb 24 [Updated 2016 Dec 22]. In: Pagon RA, Adam MP, Ardinger HH, et al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2017. [cited 1/24/2017] Available from: https://www.ncbi.nlm.nih.gov/books/NBK1352/
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
8. Hwu W, De D, Bertini E, et al. Outcomes after 1-year in presymptomatic infants with genetically diagnosed spinal muscular atrophy (SMA) treated with nusinersen: interim results from the NURTURE study. Neuromuscul Disord. 2017;27(Supplement 2):S212.
9. Finkel RS, Mercuri E, Darras BT, et al. Nusinersen versus Sham Control in Infantile-Onset Spinal Muscular Atrophy. N Engl J Med. 2017 Nov 2;377(18):1723-1732.


## History

| Date | Action and Summary of Changes |
|---|---|
| **02/12/2021** | Annual policy update |

Washington State Health Care Authority

# Spinal Muscular Atrophy Agents

**Please provide the information below, please print your answer, attach supporting documentation, sign, date, and return to our office as soon as possible to expedite this request. Without this information, we may deny the request in seven (7) working days.**

| | | | |
|---|---|---|---|
| Date of request: | Reference #: | MAS: | |
| Patient | Date of birth | ProviderOne ID | |
| Pharmacy name | Pharmacy NPI | Telephone number | Fax number |
| Prescriber | Prescriber NPI | Telephone number | Fax number |
| Medication and strength | Directions for use | Qty/Days supply | |

1. Is this request for a continuation of existing therapy? ☐ Yes ☐ No
   If yes, is there documentation of disease improvement or stability demonstrated by one of the following?
   - ☐ At least one of the functional scales or motor milestones evaluated in the previous 90 days
   - ☐ Disease progression is slower than what would otherwise be expected
   - ☐ None of the above

2. Indicate patient's diagnosis:
   - ☐ Spinal muscular atrophy (SMA)
   - ☐ Other. Specify: _____

3. Does the patient have a diagnosis of Spinal muscular atrophy (SMA) and genetic test 5q13 that demonstrates one of the following?
   - ☐ Homozygous SMN1 gene deletion
   - ☐ Homozygous SMN1 gene mutation
   - ☐ Compound heterozygous SMN1 gene mutation
   - ☐ None of the above

4. Is patient symptomatic with a phenotype of SMA I, SMA II OR SMA III? ☐ Yes ☐ No

5. Will this medication be used in combination with other Spinal Muscular Atrophy Agents (i.e Evrysdi, Spinraza)?
   - ☐ Yes. Specify: _____
   - ☐ No

6. Has the patient previously been treated with Zolgensma (onasemnogene abeparvovec-xioi)? ☐ Yes ☐ No

7. Indicate which of the following functional scales were used to document baseline and current (within the last 90 days) motor function?
   - ☐ Six-minute walk test (6MWT)
     Baseline: Date taken: Current: Date taken:
   - ☐ Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND)
     Baseline: Date taken: Current: Date taken:
   - ☐ Hammersmith Infant Neurological Exam (HINE) infant and early childhood
     Baseline: Date taken: Current: Date taken:
   - ☐ Hammersmith Functional Motor Scale Expanded (HFMSE)
     Baseline: Date taken: Current: Date taken:
   - ☐ Motor function measure (MFM32)
     Baseline: Date taken: Current: Date taken:

☐ Revised upper Limb Module (RULM) Test (non-ambulatory)
Baseline: Date taken: Current: Date taken:
☐ Other. Specify: _____

8. Does the patient have documentation of any of the following (check all that apply)?
   ☐ Neurologic examination with
   Baseline: Date taken: Current: Date taken:
   ☐ Manual Muscle Test (MMT)
   Baseline: Date taken: Current: Date taken:
   ☐ Pulmonary Function Test (PFT), if appropriate
   Baseline: Date taken: Current: Date taken:
   ☐ None

9. Is the patient ambulatory?
   ☐ Yes
   ☐ No. When did the patient lose the ability to walk?

10. Does the patient require tracheostomy or invasive ventilation? ☐ Yes ☐ No

11. Indicate for the patient:
    Weight (kg): Date taken:

12. Is the medication prescribed by a provider with expertise in treating and managing SMA?
    ☐ Yes ☐ No

**Required with this request:**

- **Neurologic examination**
- **Manual Muscle Test (MMT)**
- **Pulmunary Function Test (PFT)**
- **All motor function tests**
- **Chart notes**

| Prescriber signature | Prescriber specialty | Date |
|---|---|---|
| | | |