Washington State Health Care Authority

Spinal Muscular Atrophy Agents – nusinersen (Spinraza)

Medical policy no. 74.70.00.AA-1 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.AA - 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®) may be considered medically necessary in patients who meet the criteria described in the clinical policy below. 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 or reauthorization duration. Clients new to Apple Health or new to an MCO, who are requesting regimens for continuation of therapy should be reviewed following the reauthorization criteria listed below.

Clinical policy:

Clinical Criteria	
Spinal Muscular Atrophy	Nusinersen (Spinraza®) may be covered when **ALL** of the following are met:

	1. Confirmed diagnosis of spinal muscular atrophy (SMA) defined as **ONE** of the following genetic tests of 5q13 demonstrating: a. Homozygous SMN1 gene deletion; **OR** b. Homozygous SMN1 gene mutation; **OR** c. Compound heterozygous SMN1 gene mutation; **AND** 2. Not used simultaneously with Evrysdi (risdiplam); **AND** 3. Patient has not been previously treated with Zolgensma (onasemnogene abeparvovec-xioi); **AND** 4. Completion of **ONE** or more of the following functional scales that is appropriate for patient age and motor function within the last 90 days: a. Six-Minute Walk Test (6MWT); **OR** b. Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND); **OR** c. Hammersmith Infant Neurological Exam (HINE) – infant and early childhood; **OR** d. Hammersmith Functional Motor Scale Expanded (HFMSE); **OR** e. Revised Upper Limb Module (RULM) test (non-ambulatory); **AND** 5. Baseline and annual documentation of **ALL** of the following: a. Neurologic examination; **AND** b. Manual Muscle Test (MMT); **AND** c. Pulmonary Function Test (PFT), if able; **AND** 6. Does not require permanent mechanical ventilation; **AND** 7. Prescribed by a provider specializing in the treatment of SMA If all the above criteria are met, Spinraza may be approved for **5 doses to be administered in a 7 month period.**
Criteria (Reauthorization)	Continued use of nusinersen (Spinraza®) may be authorized when **ALL** of the following are met: 1. Documentation of criteria listed in 4 and 5 above evaluated in the previous 90 days demonstrating **ONE** of the following: a. Disease **improvement** or **stability**; **OR** b. Disease progression is slower than what would otherwise be expected If all the criteria are met Spinraza may be approved for **12 months.**

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

Definitions:

Definition	
Improvement	• **HFMSE*:** At least 3 points increase in score from pretreatment baseline • **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. • **CHOP-INTEND*:** At least a 4-point increase in score from the pretreatment baseline • **6MWT (ambulatory):** At least a 30-meter increase from pretreatment baseline • **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
10/15/2021	New policy number assigned

Washington State Health Care Authority

Spinal Muscular Atrophy Agent

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 annual functional scales and motor milestones that has been evaluated in the previous 90 days and show one of the following? (check all that apply):
     - ☐ Disease improvement or stability
     - ☐ Disease progression is slower than what would otherwise be expected
     - ☐ None of the above

2. For continuation of therapy request, what was the start date for treatment? _____

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

4. Does the patient have a confirmed 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

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) – **[Evrysdi requests only]**
     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. Does the patient require permanent mechanical ventilation? ☐ Yes ☐ No

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

11. Is the medication prescribed by a provider specializing in treatment of SMA?
☐ Yes ☐ No

Required with this request:

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

Prescriber signature	Prescriber specialty	Date