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. Baseline and annual documentation of motor function including 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. Motor Function Measure 32 (MFM32); **OR** f. 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), if appropriate; **OR** c. Pulmonary Function Test (PFT), if able; **AND** 6. Patient demonstrates ability to maintain meaningful function including, but not limited to: a. Breathing independently of permanent mechanical ventilation; **OR** b. Either ambulatory or can independently operate wheelchair; **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	• **6MWT (ambulatory):** At least a 30-meter increase from pretreatment baseline • **CHOP-INTEND*:** At least a 4-point increase in score from the pretreatment baseline • **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. • **MFM32****: At least a 30-meter increase from pretreatment baseline • **RULM (non-ambulatory):** At least a 2-point increase in score from the pretreatment baseline
Permanent Mechanical Ventilation	Tracheostomy or ≥16 hours of noninvasive ventilation per day or intubation for ≥21 consecutive days in the absence of, or following the resolution of, an acute reversible event)
Stability	The functional scale score did not worsen from baseline

*Improvement is based on minimal clinically important difference in Sprinraza clinical trials
**Improved is based on minimally clinically important difference in Evrysdi 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
12/15/2021	Approved by DUR Board