# Nusinersen

## Adjudication Guideline

| Rule Category: | Ref: No: | Version Control: | Effective Date: | Revision Date: |
|---|---|---|---|---|
| Pharmaceutical | 2022-PH-13 | Version No. V3.0 | 15th September 2022 | 29/08/2025 |

| Approved by: | Responsible: | Related Adjudication Guidelines: |
|---|---|---|
| Daman | Medical Standards & Research | |

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# 1. Abstract

## 1.1 For Members

Nusinersen is an antisense oligonucleotide drug approved by the FDA for the treatment of spinal muscular atrophy.

Spinal muscular atrophy (SMA) is a genetic (inherited) neuromuscular disease that causes muscles to become weak and waste away.

Nusinersen is available in injection form and is administered intrathecal, or an injection into the fluid of the spine.

## 1.2 For Medical Professionals

Nusinersen is an antisense oligonucleotide indicated for the treatment of spinal muscular atrophy (SMA) in pediatric and adult patients.

Spinal muscular atrophy (SMA) is a genetic (inherited) neuromuscular disease that causes muscles to become weak and waste away.

People with SMA are either missing part of the SMN1 gene on chromosome 5q or have a changed (mutated) gene. A healthy SMN1 gene produces SMN protein. People with SMA don't make enough SMN protein and lose a specific type of nerve cell in the spinal cord (called motor neurons) that control muscle movements.

Nusinersen is an intrathecal injection, or an injection into the fluid of the spine, by a healthcare professional experienced in performing lumbar punctures.

# 2. Scope

This Adjudication Rule highlights the medical indications and coverage requirements of Nusinersen for spinal muscular atrophy for all health insurance plans administered by Daman as per policy terms and conditions.

**Medical Indications:**
Nusinersen is an antisense oligonucleotide indicated for the treatment of spinal muscular atrophy (SMA) in pediatric and adult patients.

**Different Types of SMA**

**Type 1 SMA (young babies)**
is the most common and severe form of SMA. It's sometimes called Werdnig-Hoffmann disease or infantile-onset SMA and it typically presents after birth but before age six months.

**Type 2 SMA** (older babies and toddlers)
is an intermediate form of SMA and shows symptoms when they're 6 to 18 months old.

**Type 3 SMA** (children and young adults)
is a milder form of SMA. It's also known as Kugelberg-Welander disease, and its Onset usually occurs between the age of 18 months and adulthood.

**Type 4 SMA** (adults)
is very rare. It usually starts in young adulthood and causes mild motor impairment.

**Type 0 SMA** (prenatal onset)

A very severe form of SMA, prenatal onset and is usually associated with early death from respiratory failure.

A pre-symptomatic SMA patient individual is defined as having the homozygous gene deletion or homozygous mutation, or compound heterozygous mutation of the SMN1 gene (Chromosome 5) found via pre-symptomatic testing of the patient. These patients are genetically destined to develop 5q SMA.

# 3. Adjudication Policy

## 3.1 Eligibility / Coverage Criteria

### Eligibility / Coverage Criteria

- Type I, II, III, and pre-symptomatic
- Confirmed genetic documentation of 5q SMA homozygous gene deletion or homozygous mutation, deletion, or compound heterozygous mutation.
- Age of the member is 15 years or younger at the start of the treatment.
- Must be prescribed by a neurologist.
- Must not be type 0 and type IV SMA patient.
- No permanent ventilation (≥16 hours/day for 21 consecutive days in the absence of acute reversible infection) or permanent tracheostomy status.
- Member has not received gene replacement therapy previously for SMA (Ex: Zolgensma) or received gene replacement therapy earlier but worsened in clinical status.
- Nusinersen is not prescribed concurrently with Evrysdi or Zolgensma
- Should provide one of the following assessment tools as indicated by the patient motor ability:

    1) Hammersmith Infant Neurological Exam part-2 (HINE-2) or
    2) Hammersmith Functional Motor Scale Expanded (HFSME) or
    3) Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND).
    4) Revised Upper Limb Module (RULM)

### Criteria for continuation of treatment

I. All the above criteria are met.
II. Initial evaluation should be done after 5 doses (4 loadings and 1 maintenance dose) and the member should show a positive clinical response from pre-treatment baseline to Nusinersen treatment as demonstrated by at least one of the following assessments:
    A. **HINE**: Member shows improvement of at least:
        i. 2 points horizontal kick or
        ii. 1 point on other HINE scores (Ex: rolling, crawling, sitting, standing, head control, or walking) excluding voluntary grasp.
    B. **HFSME**: improvement of at least 3 points.
    C. **CHOP INTEND**: Member should exhibit at least 4 points of improvement on this scale.
    D. **RULM**: At least a 2-point increase in score from pre-treatment baseline.
III. 12month periodic re-examination must be done.

## 3.2 Non-Coverage

Off-label uses of Nusinersen that are not an FDA-approved indication or not included in the Coverage Criteria 'section of this policy is considered experimental/investigational or not a covered benefit of this policy.

| Plan | Coverage |
|---|---|
| Visitor plan | Not covered |
| Basic plan | Not covered |
| Enhanced plan | Covered as per SOB |
| Thiqa | Covered |

| Eligible clinician specialty |
|---|
| Neurosurgery |
| Neurology |
| Pediatric Neurology |
| Neurological Surgery |
| Clinical Neurophysiology |
| Pediatric Neurology/ Clinical Neurophysiology |

## 3.3 Payment and Coding Rules

Kindly apply DOH payment rules and regulations and relevant coding manuals for ICD, Drugs.

# 4. Denial Codes

| Code | Code Description |
|---|---|
| MNEC-003 | Service is not clinically indicated based on good clinical practice |
| MNEC-005 | Service/ supply may be appropriate, but too frequent. |
| CODE-010 | Activity/diagnosis inconsistent with clinician specialty |
| NCOV-003 | Service(s) is (are) not covered |
| Auth-001 | Prior approval is required and was not obtained |

Kindly use the below Pre-Approval Form for Authorization:
https://www.damanhealth.ae/main/pdf/support/Questionnaire/NUSIRENSEN_QUESTIONNAIRE.pdf

## Spinraza Pre-Authorization Form

### MEMBER INFORMATION

- Member Name: _____
- Member Card #: _____
- Policy: _____
- Date: / / 202___
- Age: _____
- Gender: ☐ Female ☐ Male

### PROVIDER INFORMATION

- Ordering Provider Name: _____
- Ordering Clinician (ID # & Name): _____
- Performing Provider Name: _____
- Performing Clinician Specialty (ID # & Name): _____
- Referring Physician Contact No.: _____

### SERVICE REQUESTED

Principal/ Primary Diagnosis: _____ ICD-10: _____

Medication (Spinraza) Dosage: _____ Quantity: _____

Treatment initiation date_____

Requested Dosage ☐ Initial dose ☐ Maintenance dose

Age of the member at the initiation of the treatment_____

### Motor ability assessment

1) Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorder (CHOP-INTEND) score: Not applicable ☐

   Baseline score: _____ Date tested: _____; Current score: _____ Date tested: _____

2) Hammersmith Infant Neurological Exam part-2 (HINE-2) score Not applicable ☐

   Baseline score: _____ Date tested: _____; Current score: _____ Date tested: _____

3) Revised Upper Limb Module (RULM) score Not applicable ☐

   Baseline score: _____ Date tested: _____; Current score: _____ Date tested: _____

4) Hammersmith Functional Motor Scale Expanded (HFMSE) motor milestone score Not applicable ☐

   Baseline score: _____ Date tested: _____; Current score: _____ Date tested:

## Spinraza Pre-Authorization Form

### Additional information

1) Genetic test confirmation of the Spinal muscular atrophy (SMA) ☐ Yes ☐ No

2) Patient requires tracheostomy or invasive or non-invasive ventilation ≥16 hours/day for 21 consecutive days
☐ Yes ☐ No

3) Spinraza prescribed concurrently with Evrysdi or Zolgensma Yes ☐ No ☐

4) Previously treated with Zolgensma ☐ No
☐ Yes (Documented evidence of worsening clinical status)

# 5. Appendices

## 5.1 References


A. *https://www.spinraza.com/content/dam/commercial/spinraza/caregiver/en_us/pdf/spinraza-prescribing-information.pdf*
B. *https://www.ema.europa.eu/en/medicines/human/EPAR/spinraza*
C. *https://www.nhs.uk/conditions/spinal-muscular-atrophy-sma/types/*
D. *https://www.uptodate.com/contents/spinal-muscular-atrophy*
E. *https://www.nhs.uk/conditions/spinal-muscular-atrophy-sma/diagnosis/*
F. *Spinraza 12 mg solution for injection - Summary of Product Characteristics (SmPC) - (emc) (medicines.org.uk)*
G. *https://www.nice.org.uk/guidance/ta588/resources/managed-access-agreement-july-2019-pdf-6842812573https://www.nice.org.uk/guidance/ta588/resources/managed-access-agreement-july-2019-pdf-6842812573*
H. *https://www.spinraza-hcp.com/content/dam/commercial/spinraza/hcp/en_us/pdf/mobility-and-physical-abilit*
I. *https://www.accessdata.fda.gov/drugsatfda_docs/label/2023/209531s011lbl.pdf*
J. *https://www.accessdata.fda.gov/drugsatfda_docs/label/2024/209531s013s014lbl.pdf*


## 5.2 Revision History

| Date | Change(s) |
|---|---|
| 15/8/2022 | Release of V1.0 |
| 23/5/2023 | Updated: questionnaire link |
| 16/08/2024 | AR review |
| 30/10/2024 | No changes/ updated in the new AR format |
| 29/08/2025 | AR Review |