

Nusirensen

Adjudication Guideline

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Pharmaceutical

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**Related Adjudication
Guidelines:**N/A

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1. Abstract

1.1 For Members

Nusirensen is an Antisense 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.

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

1.2 For Medical Professionals

Nusirensen 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.

Nusirensen 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 Nusirensen for spinal muscular atrophy for all health insurance plans administered by Daman as per policy terms and conditions.

Medical Indications:

Nusirensen 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.
- Nusirensen 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 nusirensen 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 Nusirensen 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

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-6842812573><https://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

5.2 Revision History

Date	Version No.	Change(s)
15/8/2022	V1.0	Release of V1.0
23/5/2023	V2.0	Updated: questionnaire link
16/08/2024	V3.0	AR review
30/10/2024	V4.0	No changes/ Release of V4.0