

Spinraza® (nusinersen)

When requesting Spinraza® (nusinersen), the individual requiring treatment must be diagnosed with the following FDA-approved indication and meet the specific coverage guidelines and applicable safety criteria for the covered indication.

FDA-Approved Indication

Spinraza is indicated for the treatment of spinal muscular atrophy (SMA) in pediatric and adult patients.

Coverage Guidelines

Treatment of spinal muscular atrophy (SMA)

An individual must meet **all** of the following criteria for initial authorization:

- Has had a genetic test confirming the diagnosis of SMA with bi-allelic pathogenic variants in the survival motor neuron 1 (SMN1) gene (e.g., homozygous deletion, compound heterozygous mutation, a variety of other rare mutations) *[documentation required]*;
- Has two or three survival motor neuron 2 (SMN2) gene copies OR has four SMN2 gene copies with symptoms consistent with Types 1, 2, or 3 SMA *[documentation required]*;
- Baseline motor ability assessment that suggests SMA (based on age, motor ability, and development) is provided from one of the following exams *[documentation required]*:
 - o Bayley Scales of Infant and Toddler Development;
 - o Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP-INTEND);
 - o Hammersmith Functional Motor Scale Expanded (HFMSE);
 - o Hammersmith Infant Neurological Exam Part 2 (HINE-2);
 - o Motor Function Measure-32 Items (MFM-32);
 - o Revised Upper Limb Module (RULM) test;
 - o World Health Organization motor milestone scale;
- Has not received Zolgensma (onasemnogene abeparvovec-xioi intravenous infusion) or Itivisma (onasemnogene abeparvovec-brve intrathecal injection) in the past;
- Spinraza is prescribed by or in consultation with a physician who specializes in the management of spinal muscular atrophy and/or neuromuscular disorders.

An individual must meet all of the following criteria for re-authorization:

- Has had a genetic test confirming the diagnosis of SMA with bi-allelic pathogenic variants in the survival motor neuron 1 (SMN1) gene (e.g., homozygous deletion, compound heterozygous mutation, a variety of other rare mutations);
- Has two or three survival motor neuron 2 (SMN2) gene copies OR has four SMN2 gene copies with symptoms consistent with Types 1, 2, or 3 SMA;
- Spinraza is prescribed by or in consultation with a physician who specializes in the management of spinal muscular atrophy and/or neuromuscular disorders;
- Each Spinraza dose is to be administered once every four months as maintenance therapy;
- Must meet one of the following:
 - Has experienced a positive clinical response (e.g., improvement or stabilization) from pretreatment baseline from one of the following exams [documentation required]:
 - Bayley Scales of Infant and Toddler Development;
 - Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP-INTEND);
 - Hammersmith Functional Motor Scale Expanded (HFMSE);
 - Hammersmith Infant Neurological Exam Part 2 (HINE-2);
 - Motor Function Measure-32 Items (MFM-32);
 - Revised Upper Limb Module (RULM) test;
 - World Health Organization motor milestone scale; OR
 - Has responded to Spinraza therapy and continues to benefit from ongoing Spinraza therapy by the most recent physician monitoring/assessment tools (e.g., decrease in the frequency of respiratory infections or complications, reduced need for respiratory support, prevention of permanent assisted ventilation, pulmonary function tests showing improvement, and/or bulbar function test results suggest benefits) [documentation required].

Approval duration (initial): 3 months

Approval duration (renewal): 1 year

Dosing Recommendation

Spinraza is administered intrathecally.

Low-Dose Regimen: Initially give 12 mg intrathecally as four loading doses of which the first three loading doses should be given at 14-day intervals and the fourth loading dose should be given 30 days after the third dose; The maintenance dose is 12 mg intrathecally once every 4 months and is started 4 months after the last loading dose;

Transitioning from the Low-Dose Regimen to the High-Dose Regimen: Approve one 50 mg intrathecal dose given at least 4 months after the last 12 mg maintenance dose; The maintenance dose is 12 mg intrathecally once every 4 months and is started 4 months after the last loading dose;

High-Dose Regimen: The initial dose is 50 mg intrathecally followed by a second 50 mg dose 14 days later; The maintenance dose is 28 mg intrathecally once every 4 months and is started 4 months after the last loading dose

References

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