

Spinraza® (nusinersen)

When requesting Spinraza® (nusinersen), the individual requiring treatment must be diagnosed with an 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).

Coverage Guidelines

Treatment of spinal muscular atrophy (SMA)

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

- Has had a genetic test confirming the diagnosis of SMA with bi-allelic mutations in the *survival motor neuron 1 (SMN1)* gene reported as at least one of the following: homozygous deletion, homozygous mutation, or compound heterozygous mutation;
- Has 2 or 3 survival motor neuron 2 (SMN2) gene copies OR has 4 or more SMN2 gene copies with symptoms consistent with Types 1, 2, or 3 SMA;
- Baseline motor ability assessment that suggests SMA (based on age, motor ability, and development) is provided from one of the following exams:
 - o Bayley Scales of Infant and Toddler Development, Third Edition (BSID-III);
 - 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 in the past;
- For a patient currently receiving or who has received prior treatment with Evrysdi (risdiplam oral solution), further therapy with Evrysdi will be discontinued;
- The following laboratory tests will be evaluated prior to the administration of Spinraza: prothrombin time and/or activated partial thromboplastin time, platelet count, and quantitative spot urine protein test; AND
- Spinraza is prescribed by or in consultation with a physician who specializes in the management of spinal muscular atrophy and/or neuromuscular disorders.

For reauthorization, an individual must meet all of the above **and** the following criteria:

- Four months has elapsed since the last dose;

- Has experienced a positive clinical response (e.g., improvement or stabilization) from pretreatment baseline from one of the following exams:
 - o Bayley Scales of Infant and Toddler Development, Third Edition (BSID-III);
 - 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; OR
- Has responded to Spinraza therapy and continues to benefit from ongoing Spinraza therapy by the most recent (within the past 4 months) 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).

Approval duration (initial): 3 months

Approval duration (renewal): 4 months (one dose)

Dosing Recommendation

Spinraza is administered intrathecally.

The recommended dose is 12 mg (5mL) per administration. Initiate Spinraza treatment with 4 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 3^d dose. A maintenance dose of 12mg should be administered once every 4 months thereafter.

References

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