

**CRITERIA FOR PRIOR AUTHORIZATION**

Spinraza® (nusinersen)

**PROVIDER GROUP** Professional

**MANUAL GUIDELINES** The following drugs requires prior authorization:  
Nusinersen (Spinraza®)

**CRITERIA FOR INITIAL APPROVAL** (Must meet the following criteria):

- Patient must have a diagnosis of spinal muscular atrophy (SMA), confirmed by SMN1 (chromosome 5q) gene mutation or deletion (one of the following):
  - Homozygous SMN1 gene deletion or mutation
  - Compound heterozygous SMN1 mutation
- Provider must submit documentation the patient has a sufficient number of copies of SMN2 gene defined as one of the following genetic tests demonstrating:
  - Genetic testing confirming at least 2 copies of SMN2 gene, OR
  - SMA associated symptoms before 6 months of age
- Prescribed by or in consultation with a neurologist with expertise in the diagnosis of SMA
- Prescriber must submit baseline documentation of one of the following:
  - Hammersmith Infant Neurological Exam (HINE) (infant to early childhood)
  - Hammersmith Functional Motor Scale Expanded (HFMSE)
  - Upper Limb Module (ULM) Test (Non ambulatory)
  - Children’s Hospital of Philadelphia Infant Test of Neuromuscular Disorders
- Prescriber must submit documentation of the following baseline results:
  - Platelet count
  - Coagulation laboratory testing (prothrombin time; activated partial thromboplastin time)
  - Quantitative spot urine protein testing
- Must be administered by, or under the direction of, healthcare professionals experienced in performing lumbar punctures
- Dosing must not exceed 12 mg. There are 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 3rd dose.

**LENGTH OF INITIAL APPROVAL** 6 months (1 loading dose [4 injections] and 1 maintenance dose)

PA Criteria

**CRITERIA FOR RENEWAL APPROVAL (Must meet the following criteria):**

- Patient must have a diagnosis of spinal muscular atrophy (SMA), confirmed by SMN1 (chromosome 5q) gene mutation or deletion (one of the following):
  - Homozygous SMN1 gene deletion or mutation
  - Compound heterozygous SMN1 mutation
- Provider must submit documentation the patient has a sufficient number of copies of SMN2 gene defined as one of the following genetic tests demonstrating:
  - Genetic testing confirming at least 2 copies of SMN2 gene, OR
  - SMA associated symptoms before 6 months of age
- Prescribed by or in consultation with a neurologist with expertise in the diagnosis of SMA
- Prescriber must submit post-treatment documentation with the most recent results (< 1 month prior to request) documenting a positive clinical response from pretreatment baseline status demonstrated by at least one of the following:
  - Hammersmith Infant Neurological Exam (HINE) (infant to early childhood):
    - Improvement or maintenance of previous improvement of at least 2 point (or maximal score) increase in ability to kick or Improvement or maintenance of previous improvement of at least 1 point increase in any other HINE milestone (e.g., head control, rolling, sitting, crawling, etc.), excluding voluntary grasp, AND
    - The patient exhibited improvement, or maintenance of previous improvement in more HINE motor milestones than worsening, from pretreatment baseline (net positive improvement) or Achieved and maintained any new motor milestones when they would otherwise be unexpected to do so (e.g., sit unassisted, stand, walk)
  - Hammersmith Functional Motor Scale Expanded (HFMSE):
    - Improvement or maintenance of previous improvement of at least a 3 point increase in score from pretreatment baseline or Patient has achieved and maintained any new motor milestone from pretreatment baseline when they would otherwise be unexpected to do so
  - Upper Limb Module (ULM) Test (Non ambulatory)
    - Improvement or maintenance of previous improvement of at least a 2 point increase in score from pretreatment baseline or Patient has achieved and maintained any new motor milestone from pretreatment baseline when they would otherwise be unexpected to do so
  - Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders
    - Improvement or maintenance of previous improvement of at least a 4 point increase in score from pretreatment baseline or Patient has achieved and maintained any new motor milestone from pretreatment baseline when they would otherwise be unexpected to do so
- Prescriber must submit documentation of the following has been obtained prior to each dose:
  - Platelet count
  - Coagulation laboratory testing (prothrombin time; activated partial thromboplastin time)
  - Quantitative spot urine protein testing
- Must be administered by, or under the direction of, healthcare professionals experienced in performing lumbar punctures
- Dosing must not exceed 12 mg. A maintenance dose should be administered once every 4 months.

**LENGTH OF RENEWAL APPROVAL**      12 months (3 maintenance doses)

PA Criteria

Notes:

- The mutation or deletion of genes in chromosome 5q resulting in one of the following:
  - Homozygous gene deletion or mutation (e.g., homozygous deletion of exon 7 at locus 5q13)
  - Compound heterozygous mutation (e.g., deletion of SMN1 exon 7[allele 1] and mutation of SMN1 [allele 2])
- Spinraza is unproven and not medically necessary for:
  - Other forms of SMA caused by mutations atrophy without chromosome 5q mutations or deletions or in genes other than the SMN1 gene such as: spinal muscular atrophy respiratory distress (SMARD), distal SMA, Kennedy's Disease
- Spinraza is excreted in the kidney and 33-69% of patients in clinical trials experienced elevated urine protein. For urinary protein concentration greater than 0.2 g/L, consider repeat testing and further evaluation. Coagulation abnormalities and thrombocytopenia, including acute severe thrombocytopenia have been observed. Because of the risk of thrombocytopenia and coagulation abnormalities, patients may be at increased risk of bleeding complications. Perform a platelet count and coagulation laboratory testing at baseline and prior to each administration and as clinically needed.

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DRUG UTILIZATION REVIEW COMMITTEE CHAIR

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PHARMACY PROGRAM MANAGER  
DIVISION OF HEALTH CARE FINANCE  
KANSAS DEPARTMENT OF HEALTH AND ENVIRONMENT

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