

Prior Authorization Criteria - Spinraza (nusinersen)

Approval Criteria

Initial 6-Month Approval Criteria

1. Participant has diagnosis of spinal muscular atrophy (SMA), confirmed with documentation showing:
 - a. The mutation or deletion of SMN1 genes in chromosome 5q resulting in one of the following:
 - i. Homozygous SMN1 gene deletion or mutation (e.g. homozygous deletion of exon 7 at locus 5q13).

OR

 - ii. Compound heterozygous mutation of SMN1 (e.g. deletion of SMN1 exon 7 [allele 1] and mutation of SMN1 [allele 2]).

AND

 - b. Participant has ≤ 3 copies of the SMN2 gene.
 - c. If participant is symptomatic and has > 3 copies of the SMN2 gene, request will be considered on a case-by-case basis.
2. Prescriber is board certified in one of the following adult or pediatric specialties or subspecialties: neurology, pulmonology, orthopedics, neonatal-perinatal medicine, clinical genetics and genomics, physical medicine and rehabilitation, neuromuscular medicine, or neurodevelopmental disabilities
3. Participant does not have a tracheostomy and is not ventilator dependent.
4. Documentation of ≥ 1 baseline motor milestone exam, appropriate for participant age and motor function, is submitted:
 - a. Hammersmith Infant Neurological Exam (HINE) = Infant to early childhood
 - b. Hammersmith Functional Motor Scale Expanded (HFMSSE)
 - c. Upper Limb Module (ULM) = non-ambulatory
 - d. Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND)
 - e. 6-Minute Walk Test
5. Prescriber provides documentation of baseline platelet count, prothrombin time, activated partial thromboplastin time and quantitative spot urine protein testing within the last 30 days before the request and agrees to perform these tests prior to each dose.
6. Prescriber provides goals of therapy.

Renewal Criteria

Additional approvals may be granted for 6 months (allows for 2 doses at 4 month intervals) at a time if the following criteria continue to be met:

1. Adherence to Spinraza dosing regimen, as specified in the FDA-approved labeling, is confirmed through claims for services or submission of progress notes. Repeated nonadherence may result in denial of renewal request.
2. Participant has not received a tracheostomy or become ventilator dependent.
3. Submission of ≥ 1 recent motor milestone exams, appropriate for participant age and motor function, documenting improvement from pretreatment baseline results:
 - a. **HINE Milestones:** (maximum score = 24)
 - i. One of the following:
 1. Improvement or maintenance of previous improvement of ≥ 2 point increase in ability to kick
OR
 2. Improvement or maintenance of previous improvement of ≥ 1 point in other HINE milestone (e.g. head control, rolling, sitting, crawling, etc.), excluding voluntary grasp
 - AND
 - ii. One of the following:
 1. Improvement or maintenance of previous improvement in more HINE motor milestones than worsening, from pretreatment baseline (net positive improvement)
 2. Achieved and maintained any unexpected new motor milestones (e.g. sit unassisted, stand, walk).
 - b. **HFMSE:** (maximum score = 66)
 - i. One of the following
 1. Improvement or maintenance of previous improvement of ≥ 3 point increase from pretreatment baseline
 2. Achieved and maintained any unexpected new motor milestones (e.g. sit unassisted, stand, walk).
 - c. **ULM:** (maximum score = 18 in non-ambulatory patients)
 - i. One of the following:
 1. Improvement or maintenance of previous improvement of ≥ 2 -point increase in score from pretreatment baseline.
 2. Achieved and maintained any unexpected new motor milestones (e.g. sit unassisted, stand, walk).

d. CHOP INTEND:

i. One of the following:

1. Improvement or maintenance of previous improvement of ≥ 4 point increase in score from pretreatment baseline.
2. Achieved and maintained any unexpected new motor milestones (e.g. sit unassisted, stand, walk).

e. 6-Minute Walk Test:

1. Improvement or maintenance of baseline distance
4. Documentation confirming platelet count, prothrombin time, activated partial thromboplastin time and quantitative spot urine protein testing prior to each dose is submitted.