

Medical Policy

Spinal Muscular Atrophy

Last review date: **1/1/2026**

Prior Effective Date: **10/1/2025**

Applicable Products

Spinraza (nusinersen)

Zolgensma (onasemnogene abeparvovec-xioi)

Initial Approval Criteria

Coverage may be approved if all of the following are met:

- Drug-specific criteria; **AND**
- If applicable: Trial and failure, intolerance, or a contraindication to the preferred products as listed in the medical drug list

Spinraza

- Spinraza is prescribed by a neurologist or neuromuscular specialist with expertise in the treatment of SMA; **AND**
- One of the following:
 - Individual has had SMA diagnostic test results confirming 0 copies of SMN1; **OR**
 - Molecular genetic testing of 5q SMA for one of the following: homozygous gene deletion, homozygous conversion mutation, compound heterozygote; **AND**
- Patient has not previously received gene therapy and will not concomitantly receive Zolgensma; **AND**
- Patient must not have advanced disease (complete limb paralysis, permanent ventilation support, etc.); **AND**
- Patient must have the following laboratory tests at baseline and prior to each administration:
 - Platelet count
 - Prothrombin time
 - Activated partial thromboplastin time
 - Quantitative spot urine protein testing

Zolgensma

- Patient has had a genetic test confirming the diagnosis of spinal muscular atrophy with bi-allelic pathogenic variants in the survival motor neuron 1 (SMN1) gene; **AND**
- Zolgensma is prescribed by a neurologist or neuromuscular specialist with expertise in the treatment of SMA; **AND**
- Diagnosis of SMA by a neurologist with expertise in the diagnosis of SMA; **AND**
- Patient has 4 copies or less of SMN2 gene; **AND**

- One of the following:
 - Patient is less than 2 years of age; **OR**
 - For use in a neonatal patient born prematurely, the full-term gestational age has been reached; **AND**
- Patient does not have advanced SMA (i.e., Invasive ventilation or tracheostomy, complete limb paralysis); **AND**
- Patient will not receive routine concomitant SMN modifying therapy (e.g., Spinraza); **AND**
- Physician attests that the patient will not receive Zolgensma if the most recent pre-treatment anti-AAV9 antibody titer is above 1:50; **AND**
- The following laboratory tests will be evaluated prior to administration of Zolgensma:
 - Liver function tests (normal clinical exam, total bilirubin, and prothrombin results, and ALT and AST levels below $2 \times$ ULN); **AND**
 - Complete blood count, including platelet counts; **AND**
 - Patient has undergone a renal function assessment within the last 30 days

Renewal Criteria

- None for Zolgensma
- Spinraza:
 - Patient continues to meet Initial Approval Criteria; **AND**
 - Absence of unacceptable toxicity

Length of Authorization

Zolgensma - **1 dose per lifetime**

Spinraza – **6 months**

This policy is designed to address medical guidelines that are appropriate for the majority of individuals with a particular disease, illness, or condition. Each person's unique clinical or other circumstances may warrant individual consideration, based on review of applicable medical records, as well as other regulatory, contractual and/or legal requirements.

Medical policies do not constitute medical advice, nor are they intended to govern the practice of medicine. They are intended to reflect reimbursement and coverage guidelines. Coverage for services may vary for individual members, based on the terms of the benefit contract.