Medical Policy:
Spinal Muscular Atrophy

Last review date: 6/15/2024

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 × ULN); **AND**
    - Complete blood count, including platelet counts; **AND**
    - Patient has undergone a renal function assessment within the last 30 days and has a creatinine level < 1.0 mg/dL

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