

Montana Healthcare Programs
Physician Administered Drug Coverage Criteria

ZOLGENSMA[®] (onasemnogene abeparvovec-xioi)

Updated 2/3/2021

I. Medication Description

ZOLGENSMA[®] is an adeno-associated virus vector-based gene therapy indicated for:

- Treatment of pediatric patients less than 2 years of age with spinal muscular atrophy (SMA) with bi-allelic mutations in the survival motor neuron 1 (SMN1) gene.

II. Position Statement

Coverage is determined through a prior authorization process **that must include** supporting clinical documentation for each request.

III. Initial Coverage Criteria

Member must meet all the following criteria:

- Member is less than 2 years of age with Spinal Muscular Atrophy (SMA).
- Member has reached full-term gestational age.
- Genetic testing has confirmed bi-allelic SMN1 gene deletions or dysfunctional point mutations.
- Genetic testing has confirmed ≤ 3 copies of the SMN2 gene, or member has >3 copies of the SMN2 gene with clinical symptoms consistent with SMA before 2 years of age.
- Provider must submit documentation of a baseline motor function milestone evaluation test using an age-appropriate screening tool (e.g., CHOP-INTEND).
- Member does not have complete limb paralysis or permanent ventilator dependence.
- Medication is prescribed by a neurology specialist.
- Member has baseline anti-AAV9 antibody titer of $\leq 1:50$.
- Member does not have an active viral infection.
- Baseline liver function tests, platelet counts, and troponin-1 have been performed and will continue to be assessed after treatment for at least 3 months until they return to baseline.
- Member has not previously received ZOLGENSMA[®].
- Therapy with SPINRAZA[®] or EVRYSDI[™], if applicable, will be discontinued.

IV. Renewal Coverage Criteria

ZOLGENSMA[®] is only indicated for one infusion per lifetime. The safety and effectiveness of repeat administration of ZOLGENSMA[®] has not been evaluated.

V. Quantity Limitations

Max of one 1.1×10^{14} vector genomes/kg IV as a single weight-appropriate dose per lifetime.

VI. Coverage Duration

Initial approval duration: one single infusion

Renewal approval duration: N/A