Montana Healthcare Programs
Physician-Administered Drug Coverage Criteria

ZOLGENSMA® (onasemnogene abeparvovec-xioi)

I. **Medication Description**
Zolgensma® is an adeno-associated virus vector-based gene therapy indicated for:
- Treatment of pediatric patients less than two 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 two years of age.
- 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 two 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 ≤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 three 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 $1.1 \times 10^{14}$ 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