Overview
Zolgensma is an adeno-associated virus vector-based gene therapy indicated for the 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.

Criteria
1. Criteria for Approval (The member must meet all of the following requirements):
   • Member has confirmed and symptomatic genetic diagnosis documented by bi-allelic mutations in the SMN1 gene AND three or less copies of SMN2 gene
   • Member has an anti-adeno-associated viral vector, serotype 9 (AAV9) antibody titer less than or equal to 1:50
   • Member is less than 2 years of age
   • Member has not previously received Zolgensma
   • Member does not have concomitant illness such as severe kidney or liver disease, active viral infection, or symptomatic cardiomyopathy
   • If the member is receiving treatment with Spinraza, that treatment will be discontinued

2. Dosing and Administration
   • Member will receive a single-dose Zolgensma intravenously infusion within accordance of the FDA approved labeling; 1.1 x 10^14 vector genomes (vg) per kilogram of body weight.

3. Duration of Therapy
   • Single-dose one-time intravenous infusion per lifetime

4. Exclusions
   • The member has advanced SMA as evidenced but not limited to complete paralysis of limbs, invasive ventilatory support (tracheostomy), or use of non-invasive respiratory support for more than 16 hours per day.

Effective
December 2019: Effective date.

References
