Medical Policy
Zolgensma (Onasemnogene Abeparvovec)

	Commercial and Qualified Health Plans	MassHealth
Authorization required	X	X
Authorization not required		

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 (Commercial)

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.

Criteria (MassHealth)

For its MassHealth members AllWays Health Partners follows the Zolgensma clinical guidelines as set forth in MassHealth's Drug Utilization Review Program.

1. Criteria for Approval (The member must meet **all** of the following requirements as outlined in documentation provided by the treating prescriber):
    - Appropriate diagnosis (Type 1, 2 or 3 SMA)

- A genetic test confirming the member has a diagnosis of bi-allelic mutation in the SMA1 gene (e.g. SMN1 homozygous gene deletion or mutation or compound heterozygous mutation)
- A genetic test confirming the member has two or three copies of the 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

2. Dosing and Administration
   - The prescriber is a neuromuscular specialist
   - Member will receive a single-dose Zolgensma intravenously infusion within accordance of the FDA approved labeling; 1.1×10^{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 (endotracheal tube or tracheotomy tube) or use of non-invasive respiratory assistance for at least 14 days for at least 16 hours per day.

Effective

April 1, 2020: Updated table and added Criteria section to reflect MassHealth coverage.
December 2019: Effective date.

References

Al-Zaidy S, Pickard AS, Kotha K, et al. Health outcomes in spinal muscular atrophy type 1 following AVXS-101 gene replacement therapy. Pediatric Pulmonology 2018;54:179–185.

Mendell JR, Al-Zaidy S, Shell R, et al. Single-dose gene-replacement therapy for spinal muscular atrophy. N Engl J Med. 2017;377:1713-22

Mendell JR, Al-Zaidy S, Shell R, et al. AVXS-101 Phase 1 gene-replacement therapy clinical trial in SMA type 1: 24-month event-free survival and achievement of developmental milestones. Poster presented at: The 23rd International Annual Congress of the World Muscle Society, Mendoza, Argentina, October 2–6, 2018.

Zolgensma [package insert]. Bannockburn, IL; AveXis, Inc. May 2019.