PHARMACY POLICY STATEMENT
TRICARE

DRUG NAME	Zolgensma (onasemnogene abeparvovec-xioi)
BENEFIT TYPE	Medical
STATUS	Prior Authorization Required

Zolgensma is an adeno-associated virus (AAV) vector gene therapy initially approved by the FDA in 2019. It is 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 (SMN1) gene.
Spinal muscular atrophy (SMA) is a genetic, autosomal recessive neuromuscular disorder caused by a defect in the survival of the motor neuron 1 (*SMN1*) gene. SMA is the leading genetic cause of infant mortality and affects approximately 1 in every 10,000 infants. There are multiple types of SMA, and the age of onset and severity of the disease varies with each type.

Zolgensma (onasemnogene abeparvovec-xioi) will be considered for coverage when the following criteria are met:

Spinal Muscular Atrophy (SMA)

For **initial** authorization:

1. Member is less than two years of age; OR
2. If member is a premature neonate, full-term gestational age has been reached; AND
3. Medication must be prescribed by or in consultation with a neurologist; AND
4. Member has a diagnosis of SMA confirmed by genetic testing showing **ONE** of the following:
   a) Homozygous gene deletion of the survival motor neuron 1 (SMN1) gene (e.g., absence of SMN1 gene)
   b) Homozygous mutation of the SMN1 gene (e.g., biallelic mutation of exon 7)
   c) Compound heterozygous mutation in the SMN1 gene (e.g., deletion of SMN1 exon 7 [allele 1] and mutation of SMN1 [allele 2]); AND
5. Member has documentation of 2 to 4 copies of SMN2; AND
6. Member has documentation of the following in chart notes:
   a) Liver function tests (clinical exam, AST, ALT, total bilirubin, prothrombin time);
   b) Platelet counts;
   c) Anti-AAV9 antibody titer ≤ 1:50; AND
7. Member does **NOT** have any of the following:
   a) Prior treatment with gene therapy;
   b) Active infection;
   c) Advanced SMA (e.g., complete paralysis of limbs, permanent ventilator dependence); AND
8. Medication must **NOT** be concomitantly used with Spinraza or Evrysdi (discontinuation prior to Zolgensma therapy is required).
9. **Dosage allowed:** 1.1×10^{14} vector genomes (vg) per kg of body weight.

If all the above requirements are met, the medication will be approved for 1 month.

For **reauthorization**:
1. Zolgensma will not be reauthorized.

Scenarios that do not meet the above requirements may be considers Zolgensma (onasemnogene abeparvovec-xioi) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
05/31/2019	New policy for Zolgensma created.
06/29/2020	J code updated.
05/24/2022	Transferred to a new template. Updated references. Updated age to 2 years old and younger. Updated the copy numbers to 2 to 4 copies of SMN2. Clarified SMA diagnosis. Removed childhood vaccination requirement. Added exclusion for previous Zolgensma administration and concomitant Evrysdi use. Removed baseline symptom measurement.
01/16/2026	Updated references; removed troponin-I from required documentation; replaced gestational age ≥ 35 weeks with full-term gestational age reached; removed viral from "active viral infection" in contraindication list; removed signs of aspiration from contraindication list.

References:

1. Zolgensma [prescribing information]. AveXis, Inc; 2025.
2. Lowes LP, Alfano LN, Arnold WD, et al. Impact of Age and Motor Function in a Phase 1/2A Study of Infants With SMA Type 1 Receiving Single-Dose Gene Replacement Therapy. *Pediatr Neurol*. 2019;98:39-45. doi:10.1016/j.pediatrneurol.2019.05.005
3. Kolb SJ, Coffey CS, Yankey JW, et al. Natural history of infantile-onset spinal muscular atrophy. *Ann Neurol.* 2017;82(6):883 891.
4. Govoni A, Gagliardi D, Comi GP, Corti S. Time Is Motor Neuron: Therapeutic Window and Its Correlation with Pathogenetic Mechanisms in Spinal Muscular Atrophy. *Mol Neurobiol*. 2018;55(8):6307-6318. doi:10.1007/s12035-017-0831-9
5. Glascock J, Sampson J, Connolly AM, et al. Revised Recommendations for the Treatment of Infants Diagnosed with Spinal Muscular Atrophy Via Newborn Screening Who Have 4 Copies of SMN2. *J Neuromuscul Dis*. 2020;7(2):97-100. doi:10.3233/JND-190468.
6. Strauss KA, Farrar MA, Muntoni F, et al. Onasemnogene abeparvovec for presymptomatic infants with three copies of SMN2 at risk for spinal muscular atrophy: the Phase III SPR1NT trial. *Nat Med.* 2022;28(7):1390-1397. doi:10.1038/s41591-022-01867-3
7. Strauss KA, et al. Onasemnogene abeparvovec for presymptomatic infants with two copies of SMN2 at risk for spinal muscular atrophy type 1: the Phase III SPR1NT trial. *Nat Med.* 2022. https://doi.org/10.1038/s41591-022-01866-4.
8. Schroth M, Deans J, Arya K, et al. Spinal Muscular Atrophy Update in Best Practices: Recommendations for Diagnosis Considerations. *Neurol Clin Pract.* 2024;14(4):e200310. doi:10.1212/CPJ.0000000000200310
9. Schroth MK, Deans J, Bharucha Goebel DX, et al. Spinal Muscular Atrophy Update in Best Practices: Recommendations for Treatment Considerations. *Neurol Clin Pract*. 2025;15(1):e200374. doi:10.1212/CPJ.0000000000200374
10. Glascock J, Sampson J, Haidet-Phillips A, et al. Treatment Algorithm for Infants Diagnosed with Spinal Muscular Atrophy through Newborn Screening. *J Neuromuscul Dis*. 2018;5(2):145-158. doi:10.3233/JND-180304

Effective date: 07/01/2026
Revised date: 01/16/2026