

PHARMACY POLICY STATEMENT

Mississippi Medicaid

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 $\leq 1:50$; 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.



TrueCare 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