CareSource Rxinnovations

PHARMACY POLICY STATEMENT
Arkansas PASSE

DRUG NAME	Zolgensma (onasemnogene abeparvovec-xioi)
BILLING CODE	J3399
BENEFIT TYPE	Medical
SITE OF SERVICE ALLOWED	Outpatient
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;
2. Medication must be prescribed by or in consultation with a neurologist; AND
3. Member has a diagnosis of SMA confirmed by genetic/newborn testing showing any 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])
4. Member has 2 to 4 copies of SMN2; AND
5. Member has documentation of ALL of the following in chart notes:
   a) Liver function tests (clinical exam, AST, ALT, total bilirubin, prothrombin time);
   b) Platelet counts and troponin-I;
   c) Baseline testing for the presence of anti-AAV9 antibodies (titer must be ≤ 1:50); AND
6. Member's gestational age is ≥ 35 weeks if premature neonate; AND
7. Medication must **not** be concomitantly used with Spinraza or Evrysdi (discontinuation of Spinraza and Evrysdi prior to Zolgensma therapy is required); AND
8. Member has **not** have any of the following:
   a) Signs of aspiration;
   b) Active viral infection;
   c) Advanced SMA (e.g., complete paralysis of limbs, permanent ventilator dependence)
   d) Prior treatment with Zolgensma.
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 for continuous use.

CareSource 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.

References:

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15. Strauss KA, 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. https://doi.org/10.1038/s41591-022-01867-3.

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