Humana. CareSource™

PHARMACY POLICY STATEMENT
Kentucky Medicaid

DRUG NAME	Zolgensma (onasemnogene abeparvovec-xioi)
BILLING CODE	TBD
BENEFIT TYPE	Medical
SITE OF SERVICE ALLOWED	Outpatient Hospital
COVERAGE REQUIREMENTS	Prior Authorization Required (Non-Preferred Product) QUANTITY LIMIT— 1.1×10^{14} vector genomes per kilogram (vg/kg) of body weight
LIST OF DIAGNOSES CONSIDERED **NOT** MEDICALLY NECESSARY	Click Here

Zolgensma (onasemnogene abeparvovec-xioi) is a **non-preferred** product and will only be considered for coverage under the **medical** benefit when the following criteria are met:

Members must be clinically diagnosed with one of the following disease states and meet their individual criteria as stated.

SPINAL MUSCULAR ATROPHY (SMA)

For **initial** authorization:

1. Member is less than 12 months old at the time of infusion and has documented onset of symptoms before 6 months of age; AND
2. Member has documented diagnosis of SMA type I confirmed by ALL of the following diagnostic test results (both a and b):
   a) The mutation or deletion of genes in chromosome 5q resulting in one of the following:
      i) homozygous gene deletion OR mutation (e.g., homozygous deletion of exon 7 at locus 5q13);
      ii) compound heterozygous mutation (e.g., deletion of SMN1 exon 7 (allele 1) and mutation of SMN1 (allele 2));
   b) Genetic testing confirming 2 copies of SMN2; AND
3. Medication must be prescribed by or in consultation with a neurologist; AND
4. Member does **not** have advanced SMA (e.g., complete paralysis of limbs, permanent ventilator dependence); AND
5. Medication must **not** be concomitantly used with Spinraza (discontinuation of Spinraza prior to Zolgensma therapy is required); AND
6. On day one prior to Zolgensma infusion member will receive systemic corticosteroids equivalent to oral prednisolone at 1 mg/kg of body weight per day (for a total of 30 days); AND
7. Member has documented ALL of the following:
   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
8. Member has documentation of baseline of at least one of the following exams (based on patient age and motor ability):
   a) Hammersmith Infant Neurological Exam (HINE) (infant to early childhood);

b) Hammersmith Functional Motor Scale Expanded (HFMSE);
c) Upper Limb Module (ULM) Test (Non ambulatory);
d) Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND); AND

9. Member's gestational age is ≥ 35 weeks; AND
10. Member must be up-to-date on childhood vaccinations and prophylaxis against respiratory syncytial virus; AND
11. Member has **not** have any of the following:
    a) Signs of aspiration;
    b) Active viral infection;
    c) Concomitant use of drugs for treatment of myopathy or neuropathy, agents used to treat diabetes mellitus, or ongoing immunosuppressive therapy or immunosuppressive therapy within 3 months (e.g., corticosteroids, cyclosporine, tacrolimus, methotrexate, cyclophosphamide, intravenous immunoglobulin, rituximab);
    d) Tracheostomy (i.e., invasive ventilatory support) or required of non-invasive ventilatory support while awake over the 7 days;
    e) Upper or lower respiratory infection requiring medical attention, medical intervention, or increase in supportive care of any manner within 4 weeks prior to request.
12. **Dosage allowed:** 1.1×10^{14} vector genomes (vg) per kg of body weight.

Note: Use of Zolgensma in premature neonates before reaching full term gestational age is not recommended because concomitant treatment with corticosteroids may adversely affect neurological development.

If member meets all the requirements listed above, 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 the diseases that are not listed in this document.

DATE	ACTION/DESCRIPTION
05/31/2019	New policy for Zolgensma (onasemnogene abeparvovec-xioi) created.

References:

1. Zolgensma [prescribing information]. Bannockburn, IL: AveXis, Inc; 2019.
2. AveXis, Inc. Gene Transfer Clinical Trial for Spinal Muscular Atrophy Type 1. In: ClinicalTrials.gov [Internet]. Bethesda (MD): National Library of Medicine (US). Available from: https://clinicaltrials.gov/ct2/show/NCT02122952?term=ZOLGENSMA&rank=8. Identifier: NCT02122952.
3. 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.
4. Kolb SJ, Coffey CS, Yankey JW, et al. Natural history of infantile-onset spinal muscular atrophy. Ann Neurol. 2017;82(6):883 891.
5. Govoni A, Gagliardi D, Comi GP, Corti S. Time is motor neuron: therapeutic window and its correlation with pathogenic mechanisms in spinal muscular atrophy. Mol Neurobiol. 2018;55(8):6307 6318.
6. Stifani N. Motor neurons and the generation of spinal motor neuron diversity. Front Cell Neurosci. 2014;8:293.
7. Prior TW. Perspectives and diagnostic considerations in spinal muscular atrophy. Genet Med. 2010;12(3):145 152.

8. Farrar MA, et al. Emerging therapies and challenges in spinal muscular atrophy. Ann Neurol 2017;81(3):355–368.
9. De Sanctis R, et al. Developmental milestones in type I spinal muscular atrophy. Neuromusc Disord 2016;26(11):754–759.
10. Lowes LP, 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. Pediatric Neurology (2019).
11. Waldrop MA, et al. Current Treatment Options in Neurology—SMA Therapeutics. Curr Treatment Options Beurology. 2019;21(6):25.

Effective date: 07/01/2019
Revised date: 05/31/2019