Onasemnogene abeparvovec-xioi (Zolgensma®)	Place of Service Hospital Administration Outpatient Facility Administration HCPCS: **J3399** per treatment, up to 5×10^{15} vector genomes

Condition(s) listed in policy *(see criteria for details)*

- Spinal muscular atrophy (SMA)

AHFS therapeutic class: Gene therapy
Mechanism of action: Adeno-associated viral vector-based gene therapy containing a transgene encoding the human survival motor neuron (SMN) protein.

(1) Special Instructions and Pertinent Information

Covered under the medical benefit, please submit clinical information for prior authorization review.

(2) Prior Authorization/Medical Review is required for the following condition(s)

All requests for Zolgensma® (onasemnogene abeparvovec-xioi) must be sent for clinical review and receive authorization prior to drug administration or claim payment.

Spinal muscular atrophy (SMA)

1. Prescribed by a pediatric neurologist, **AND**
2. Diagnosis of SMA confirmed by genetic testing demonstrating bi-allelic mutations in the survival motor neuron 1 (*SMN1*) gene by one of the following:
    a. Deletion of both copies of the *SMN1* gene, or
    b. Identification of pathogenic variant(s) in both copies of the *SMN1* gene

    AND
3. Patient is less than 2 years of age, **AND**
4. Genetic documentation of 3 or fewer copies of survival motor neuron 2 (*SMN2*), **AND**
5. Patient does not have advanced SMA (e.g., complete paralysis of limbs, permanent ventilator-dependence), **AND**
6. Not being used in combination with Spinraza or Evrysdi

Covered Doses:
Up to 1.1×10^{14} vector genomes (vg) per kg of body weight as a single-dose intravenous (IV) infusion.

Coverage period:
One-time treatment per lifetime

ICD-10:
G12.0, G12.1

(3) The following condition(s) DO NOT require Prior Authorization/Preservice

All requests for Zolgensma® (onasemnogene abeparvovec-xioi) must be sent for clinical review and receive authorization prior to drug administration or claim payment.

(4) This Medication is NOT medically necessary for the following condition(s):

Blue Shield's research indicates there is inadequate clinical evidence to support off-label use of this drug for the following conditions (Health and Safety Code 1367.21):

- Zolgensma for the treatment of SMA patients with 4 or more copies of SMN2 is considered investigational

Coverage for a Non-FDA approved indication, requires that criteria outlined in Health and Safety Code § 1367.21, including objective evidence of efficacy and safety are met for the proposed indication.

Please refer to the Provider Manual and User Guide for more information.

(5) Additional Information

How supplied:

- Suspension for intravenous infusion, supplied as single-use vials.

- Per prescribing information, Zolgensma is provided in a kit containing 2 to 9 vials, as a combination of 2 vial fill volumes (either 5.5 mL or 8.3 mL). All vials have a nominal concentration of 2.0×10^{13} vector genomes (vg) per mL. Each vial of Zolgensma contains an extractable volume of not less than either 5.5 mL or 8.3 mL.

(6) References

- AHFS®. Available by subscription at http://www.lexi.com
- DrugDex®. Available by subscription at http://www.micromedexsolutions.com/home/dispatch
- Zolgensma® (onasemnogene abeparvovec-xioi) [Prescribing Information]. Bannockburn, IL: Novartis Gene Therapies, Inc.; 2/2023.

(7) Policy Update

Date of last review: 4Q2023
Date of next review: 4Q2024
Changes from previous policy version:

- No clinical change to policy following routine annual review.

BSC Drug Coverage Criteria to Determine Medical Necessity
Reviewed by P&T Committee