

Onasemnogene abeparvovec-xioi  
(Zolgensma®)

Place of Service  
Hospital Administration  
Outpatient Facility Administration

HCPs: J3399 per treatment, up to 5 x 10<sup>15</sup>  
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.

**(I) 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  $1.1 \times 10^{14}$  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  $2.0 \times 10^{13}$  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*