

## onasemnogene abeparvovec-xioi (Zolgensma)

### Commercial Medical Benefit Drug Policy

#### Place of Service

Hospital Administration

Outpatient Facility Administration

#### Drug Details

**USP Category:** GENETIC OR ENZYME OR PROTEIN DISORDER: REPLACEMENT, MODIFIERS, TREATMENT

**Mechanism of Action:** Adeno-associated viral vector-based gene therapy containing a transgene encoding the human survival motor neuron (SMN) protein

#### HCPCS:

J3399:Injection, onasemnogene abeparvovec-xioi, per treatment, up to  $5 \times 10^{15}$  vector genomes

#### 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.

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

- Spinal Muscular Atrophy (SMA)

Any condition not listed in this policy requires a review to confirm it is medically necessary. For conditions that have not been approved for intended use by the Food and Drug Administration (i.e., off-label use), the criteria outlined in the Health and Safety Code section 1367.21 must be met.

#### **Special Instructions and Pertinent Information**

Provider must submit documentation (such as office chart notes, lab results or other clinical information) to ensure the member has met all medical necessity requirements.

The member's specific benefit may impact drug coverage. Other utilization management processes, and/or legal restrictions may take precedence over the application of this clinical criteria.

For billing purposes, drugs must be submitted with the drug's assigned HCPCS code (as listed in the drug policy) and the corresponding NDC (national drug code). An unlisted, unspecified, or miscellaneous code should not be used if there is a specific code assigned to the drug.

#### **Coverage Criteria**

**The following condition(s) require Prior Authorization/Preservice.**

#### **Spinal Muscular Atrophy (SMA)**

**Meets medical necessity if all the following are met:**

1. Prescribed by a pediatric neurologist
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
  - b. Identification of pathogenic variant(s) in both copies of the SMN1 gene

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

**Covered Doses:**

Not to exceed  $1.1 \times 10^{14}$  vector genomes (vg) per kg of body weight as a single-dose intravenous infusion

**Coverage Period:**

One-time treatment per lifetime

**ICD-10:**

G12.0, G12.1

**References**

1. AHFS. Available by subscription at <http://www.lexi.com>
2. DrugDex. Available by subscription at <http://www.micromedexsolutions.com/home/dispatch>
3. 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; 7:97-100.
4. Mercuri, E, Bertini, E, Iannaccone, S. Childhood spinal muscular atrophy: controversies and challenges. *Lancet Neurol* 2012; 11(5): 443-452.
5. Wang CH, Finkel RS, Bertini ES, et al. Consensus statement for standard of care in spinal muscular atrophy. *J Child Neurol* 2007; 22:1027-1049.
6. Zolgensma (onasemnogene abeparvovec-xioi) Prescribing Information. Novartis Gene Therapies Inc., Bannockburn, IL: 2/2025.

**Review History**

Date of Last Annual Review: 4Q2025

Changes from previous policy version:

- No clinical change following annual review.

*Blue Shield of California Medication Policy to Determine Medical Necessity  
Reviewed by P&T Committee*