blue california
Promise Health Plan

onasemnogene abeparvovec-xioi (Zolgensma)

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 5x10^15 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 × 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 California Code of Regulations (CCR), Title 22, Section 51303 and 51313 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.

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:

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   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 × 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) Pprescribing 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