Corporate Medical Policy: Onasemnogene abeparvovec (Zolgensma®)

Restricted Product(s):

- onasemnogene abeparvovec (Zolgensma®) intravenous infusion for administration by a healthcare professional

FDA Approved Use:

- For the treatment of patients less than 2 years of age with spinal muscular atrophy with bi-allelic mutations in the *survival motor neuron 1* (*SMN1*) gene
    - Limitations of use: Not for repeat administration or for use in advanced SMA (e.g., complete paralysis of limbs, permanent ventilator dependence)

Criteria for Medical Necessity:

The restricted product(s) may be considered medically necessary when the following criteria are met:

1. The patient is less than 2 years of age; **AND**
2. The patient has a diagnosis of **spinal muscular atrophy (SMA)** with bi-allelic mutations in the *survival motor neuron 1* (*SMN1*) gene **[medical record documentation required]**; **AND**
3. The diagnosis has been confirmed by genetic testing consisting of one of the following **[medical record documentation required]**:
    a. Homozygous deletion of *SMN1* exon 7; **OR**
    b. Compound heterozygosity for *SMN1* exon 7 deletion and small mutation; **AND**
4. The patient has four or fewer copies of the *survival motor neuron 2* (*SMN2*) gene **[medical record documentation required]**; **AND**
5. The patient does not have advanced SMA (e.g., complete paralysis of limbs, or permanent ventilator dependence defined as invasive ventilation [tracheostomy] or at least 16 hours of respiratory assistance per day continuously for at least 14 days in the absence of an acute, reversible illness or a perioperative state) **[medical record documentation required]**; **AND**
6. The patient has had laboratory testing confirming anti-adeno-associated virus serotype 9 (AAV9) antibody titer ≤ 1:50 **[medical record documentation required]**; **AND**
7. The prescriber is a board-certified neurologist or pediatric neurologist who is experienced in the diagnosis and management of SMA and practices in a research academic setting **[medical record documentation required]**; **AND**
8. For members with North Carolina benefits/coverage seeking care within North Carolina, the provider is in the Blue Premier health system network; **AND**
9. The patient has not received prior treatment with onasemnogene abeparvovec-xioi (Zolgensma®) or other gene replacement therapy for SMA **[medical record documentation required]**; **AND**

10. The patient will not be treated concurrently with risdiplam (Evrysdi™) and/or nusinersen (Spinraza®), and any existing authorizations will be closed upon approval of onasemnogene abeparvovec-xioi (Zolgensma®); **AND**
11. The requested dose is within FDA labeled dosing for the requested indication **[medical record documentation required]**.

Duration of Approval: 30 days (one-time, single-dose treatment per lifetime)

Please note, for certain identified gene and cellular therapies such as onasemnogene abeparvovec-xioi (Zolgensma®), when coverage is available and the individual meets medically necessary criteria, distribution from a specialty pharmacy provider due to cost (distribution channel restriction) may be required in order for coverage to be provided. **Please contact Blue Cross NC to coordinate this therapy.

FDA Label Reference				
Medication	**Indication**	**Dosing**	**HCPCS**	**Maximum Units***
onasemnogene abeparvovec-xioi (Zolgensma®) intravenous (IV) infusion	SMA in patients < 2 years old with bi-allelic mutations in the *SMN1* gene	Single-dose one-time IV infusion of 1.1×10^{14} vector genomes (vg) per kg of body weight without retreatment	J3399	1

***Maximum units allowed for duration of approval**

Other revenue codes that may be applicable to this policy: 0891, 0892

References: all information referenced is from FDA package insert unless otherwise noted below.

Policy Implementation/Update Information: Criteria and treatment protocols are reviewed annually by the Blue Cross NC P&T Committee, regardless of change. This policy is reviewed in Q4 annually.

April 2026: Coding change: Added the following applicable revenue codes associated with policy HCPCS code(s): 0891 (Special Processed Drugs – FDA Approved Cell Therapy) and 0892 (Special Processed Drugs – FDA Approved Gene Therapy). **Policy notification given 2/1/2026 for effective date 4/1/2026**.
November 2025: Criteria update: Minor formatting updates with no change to policy intent.
January 2023: Criteria change: Changed SMN2 gene copy requirement from three or fewer copies to four or fewer copies.
June 2021: Criteria change: Medical policy formatting change. **Policy notification given 4/16/2021 for effective date 6/16/2021**.

*Further historical criteria changes and updates available upon request from Medical Policy and/or Corporate Pharmacy.