Oscar Clinical Guideline: Zolgensma (onasemnogene abeparvovec-xioi) (CG061, Ver. 5)

Zolgensma (onasemnogene abeparvovec-xioi)

Disclaimer

Clinical guidelines are developed and adopted to establish evidence-based clinical criteria for utilization management decisions. Clinical guidelines are applicable according to policy and plan type. The Plan may delegate utilization management decisions of certain services to third parties who may develop and adopt their own clinical criteria.

Coverage of services is subject to the terms, conditions, and limitations of a member's policy, as well as applicable state and federal law. Clinical guidelines are also subject to in-force criteria such as the Centers for Medicare & Medicaid Services (CMS) national coverage determination (NCD) or local coverage determination (LCD) for Medicare Advantage plans. Please refer to the member's policy documents (e.g., Certificate/Evidence of Coverage, Schedule of Benefits, Plan Formulary) or contact the Plan to confirm coverage.

Summary

Zolgensma targets and repairs the missing or non-functioning gene that is responsible for causing spinal muscular atrophy. Zolgensma is delivered as a single-dose, intravenous gene replacement therapy for children under 2 years old. Early restoration of the human survival motor neuron gene (SMN) may prevent the loss of motor neurons and subsequent muscle degeneration. As a new gene therapy, Zolgensma does not have a long-term research study. The member's guardian should discuss potential adverse events (e.g., hepatotoxicity and sensory neuron/ganglion toxicity) with careful consideration with the member's healthcare provider. Safe product handling and risk mitigation must be enforced to administer Zolgensma due to viral vector shedding.

Definitions

"Gene therapy" is a technique that replaces a mutated gene with a healthy gene, inactivates a mutated gene, or introduces a new gene that helps fight against diseases and disorders.

"Permanent ventilation" is defined as requiring invasive ventilation (tracheostomy), or respiratory assistance for 16 or more hours per day (including noninvasive ventilatory support) continuously for 14 or more days in the absence of an acute reversible illness, excluding perioperative ventilation.

"Spinal muscular atrophy (SMA)" is a genetic disease that affects the nervous systems and voluntary muscle movement. There is a loss of motor neurons in the spinal cord that cannot send signals for the muscles to move, resulting in weak and smaller muscles.

Medical Necessity Criteria for Authorization

The Plan considers **Zolgensma (onasemnogene abeparvovec-xioi)** medically necessary when **ALL** of the following criteria are met:

1. The prescriber is a neurologist or neuromuscular specialist with expertise in the diagnosis and management of spinal muscular atrophy (SMA); ***AND***
2. The member is less than (<) 2 years of age at time of treatment; ***AND***
3. The member has body weight body weight of 13.5 kg or less at time of treatment; ***AND***
4. The member is diagnosed with autosomal recessive 5 q13-linked (genetically proven) Spinal Muscular Atrophy (SMA) with bi-allelic mutations in the survival motor neuron 1 (SMN1) gene; ***AND***
5. The member shows ability to sit independently (if age appropriate) and does NOT have complete paralysis of limbs or permanent ventilator dependence; ***AND***
6. The member has baseline anti-adeno-associated virus 9 (anti-AAV9) antibody titer ≤1:50 measured by ELISA (enzyme-linked immunosorbent assay); ***AND***
7. The following baseline laboratory testing has been conducted:
    a. liver function (clinical exam, AST, ALT, total bilirubin, prothrombin time); ***and***
    b. platelet counts; ***and***
    c. troponin-I; ***AND***
8. The requested medication will not be used concurrently with other SMA therapies such as Spinraza (nusinersen) or Evrysdi (risdiplam); ***AND***
9. The member does not have a history of prior treatment with Zolgensma (onasemnogene abeparvovec-xioi) or any other gene transfer therapy for SMA; ***AND***
10. Zolgensma (onasemnogene abeparvovec-xioi) is dosed at 1.1×10^{14} vector genomes per kilogram (vg/kg) of body weight administered as a one-time intravenous infusion.

If the above criteria are met, Zolgensma (onasemnogene abeparvovec-xioi) will be approved for one-time administration at the recommended dosing.

Please note:

1. *Approval is provided for one-time single IV infusion only, in alignment with FDA-approved labeling.*
2. *Retreatment with Zolgensma (onasemnogene abeparvovec-xioi) is considered investigational, as safety and efficacy of repeat administrations have not been clinically established.*

Experimental or Investigational / Not Medically Necessary

Zolgensma (onasemnogene abeparvovec-xioi) for any other indication is considered experimental or investigational. Non-covered indications include, but are not limited to, the following:

- Advanced SMA (i.e., complete paralysis of limbs or ventilator dependence)
- Premature infants before reaching full-term gestational age (because concomitant use of corticosteroids may adversely affect neurological development)
- Repeat administration (because the safety and effectiveness of repeat administration of ZOLGENSMA have not been evaluated)

Applicable Billing Codes (HCPCS/CPT Codes)

CPT/HCPCS Codes considered medically necessary if criteria are met:	
Code	*Description*
96365	Intravenous infusion, for therapy, prophylaxis, or diagnosis (specify substance or drug); initial, up to 1 hour
J3399	Injection, onasemnogene abeparvovec-xioi, per treatment, up to 5x10^15 vector genomes
ICD-10 codes considered medically necessary if criteria are met:	
Code	*Description*
G12.0	Infantile spinal muscular atrophy, type I [Werdnig-Hoffman]
G12.1	Other inherited spinal muscular atrophy
G12.8	Other spinal muscular atrophies and related syndromes
G12.9	Spinal muscular atrophy, unspecified

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