Oscar Clinical Guideline: Onasemnogene abeparvovec (Zolgensma [-xioi]; Itvisma, [-brve]) (CG061, Ver. 8)

Onasemnogene abeparvovec

- Zolgensma (onasemnogene abeparvovex-xioi)
- Itvisma (onasemnogene abeparvovex-brve)

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

Spinal Muscular Atrophy (SMA) is a rare, severe genetic disorder characterized by the loss of motor neurons in the spinal cord and brainstem, leading to progressive muscle weakness and atrophy. It is caused by deletions or mutations in the survival motor neuron 1 (SMN1) gene, resulting in insufficient production of the survival motor neuron (SMN) protein essential for motor neuron survival and function. The disease is classified into several types based on age of onset and severity, with Type 1 (infantile SMA or Wednig-Hoffman disease) being the most severe and common infantile form.

Zolgensma (onasemnogene abeparvovec-xioi) is a gene replacement therapy designed to address the genetic root cause of SMA. It is delivered as a one-time, intravenous infusion and uses a modified, non-replicating adeno-associated virus (AAV9) vector to deliver a functional copy of the human SMN gene to motor neuron cells. This therapy aims to restore SMN protein production, potentially halting or reversing the progression of SMA.

- Zolgensma (onasemnogene abeparvovec-xioi) is indicated for the treatment of pediatric patients less than 2 years of age with SMA with bi-allelic mutations in the SMN1 gene. It is particularly effective when administered early, ideally before the onset of symptoms, as it may prevent or significantly delay the loss of motor neurons and subsequent muscle degeneration.

Itvisma (onasemnogene abeparvovex-brve) suspension for intrathecal injection is an AAV vector-based gene therapy indicated for those with SMA in adult and pediatrics 2 years of age and older with confirmed mutation in SMN1 gene.

While both are AAV9-based gene therapies for SMA, Itvisma (onasemnogene abeparvovex-brve) is administered intrathecally (via lumbar puncture) whereas Zolgensma (onasemnogene abeparvovec-xioi) is given intravenously. Other treatment options for SMA include Spinraza (nusinersen), an intrathecally administered antisense oligonucleotide, and Evrysdi (risdiplam), an orally administered small molecule SMN2 splicing modifier. However, Zolgensma (onasemnogene abeparvovec-xioi) and Itvisma (onasemnogene abeparvovex-brve) are unique in their potential to provide a one-time treatment that addresses the genetic cause of the disease.

“[s]” indicates state mandates may apply.

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.

Clinical Indications

Medical Necessity Criteria for Initial Clinical Review

Initial Indication-Specific Criteria

Zolgensma (onasemnogene abeparvovec-xioi)

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 is diagnosed with Spinal Muscular Atrophy (SMA) with bi-allelic mutations (deletion or point mutations) in the survival motor neuron 1 (SMN1) gene; *AND*
4. The member has 1-3 copies of the SMN2 gene, confirmed by genetic testing (i.e., submission of medical records confirming the member has 3 copies or less of the SMN2 gene)*; AND*
5. The member experienced onset of disease before 6 months of age.
6. The member does not have advanced SMA (e.g. complete paralysis of limbs or permanent ventilator dependence, respiratory assistance for 16 or more hours per day [including non-invasive respiratory support] continuously for 14 or more days in the absence of acute reversible illness [excluding perioperative ventilation]); *AND*
7. The member has baseline anti-adeno-associated virus 9 (anti-AAV9) antibody titer ≤1:50 measured by ELISA (enzyme-linked immunosorbent assay); *AND*
8. The following baseline laboratory testing has been conducted and the member has been determined to be clinically stable:
    a. Liver function (clinical exam, aspartate aminotransferase [AST], alanine aminotransferase [ALT], total bilirubin, prothrombin time); *and*
    b. Complete blood count (including hemoglobin and platelet count); *and*

c. Creatinine; *and*
d. Troponin-I; *AND*

9. The member does not have an active infectious process (e.g., viral, bacterial or febrile illness) prior to treatment; *AND*
10. The member does not have a serious concomitant illness (i.e., severe liver or kidney disease, symptomatic cardiomyopathy); *AND*
11. The member's vaccination status will be up to date prior to Zolgensma (onasemnogene abeparvovec-xioi) administration; *AND*
12.
13. If the member is on Spinraza (nusinersen) or Evrysdi (risdiplam), it will be discontinued prior to the administration of the requested drug; *AND*
14. The member does not have a history of prior treatment with Zolgensma (onasemnogene abeparvovec-xioi), Itvisma (onasemnogene abeparvovec-brve), or or any other gene transfer therapy for SMA; *AND*
15. 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.

<u>If the above criteria are met, Zolgensma (onasemnogene abeparvovec-xioi) will be authorized for one dose per lifetime, with an approval duration of 6 months.</u>[5]

<u>Itvisma (onasemnogene abeparvovec-brve)</u>

The Plan considers <u>Itvisma (onasemnogene abeparvovec-brve)</u> 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 2 to less than 18 years of age at time of treatment; *AND*
3. The member is diagnosed with autosomal recessive 5q13-linked (genetically proven) Spinal Muscular Atrophy (SMA) with bi-allelic mutations (deletion or point mutations) in the survival motor neuron 1 (SMN1) gene; *AND*
4. The member has 1-3 copies of the SMN2 gene, confirmed by genetic testing (i.e., submission of medical records confirming the member has 3 copies or less of the SMN2 gene)*; AND*
5. *The member's onset of clinical signs and symptoms of disease occurred at 6 months of age or older.*
6. The member does NOT require invasive ventilation, awake noninvasive ventilation for greater than 6 hours during a 24-hour period, noninvasive ventilation for greater than 12 hours during a 24-hour period, or require tracheostomy; *AND*
7. The member has baseline anti-adeno-associated virus 9 (anti-AAV9) antibody titer ≤1:50 measured by ELISA (enzyme-linked immunosorbent assay); *AND*

8. The following baseline laboratory testing has been conducted and the member has been determined to be clinically stable:
    a. Liver function (clinical exam, aspartate aminotransferase [AST], alanine aminotransferase [ALT], total bilirubin, prothrombin time); *and*
    b. Complete blood count (including hemoglobin and platelet count); *and*
    c. Creatinine; *and*
    d. Troponin-I; and
    e. Neurologic evaluation; *AND*
9. The member does not have contraindication(s) to lumbar puncture procedure (e.g., increased intracranial pressure, any impediment to cerebrospinal fluid access, administration of any intrathecal therapy)*; AND*
10. The member does not have an active infectious process (e.g., viral, bacterial or febrile illness) prior to treatment*; AND*
11. The member does not have a serious concomitant illness (i.e., severe liver or kidney disease, symptomatic cardiomyopathy); *AND*
12. The member's vaccination status will be up to date prior to Itvisma (onasemnogene abeparvovec-brve) administration; *AND*
13. The requested medication will not be used concurrently with other SMA therapies such as Spinraza (nusinersen) or Evrysdi (risdiplam); *AND*
14. If the member is on Spinraza (nusinerse) or Evrysdi (risdiplam), it will be discontinued prior to the administration of the requested drug; *AND*
15. The member does not have a history of prior treatment with Zolgensma (onasemnogene abeparvovec-xioi), Itvisma (onasemnogene abeparvovec-brve), or any other gene transfer therapy for SMA; *AND*
16. Itvisma (onasemnogene abeparvovec-brve), is dosed at 1.2×10^{14} vector genomes administered as a one-time intrathecal bolus injection.

If the above criteria are met, Itvisma (onasemnogene abeparvovec-brve) will be authorized for one dose per lifetime, with an approval duration of 6 months.[s]

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) or Itvisma (onasemnogene abeparvovec-brve) is considered investigational, as safety and efficacy of repeat administrations have not been clinically established.*

Experimental or Investigational or Unproven / Not Medically Necessary[s]

Zolgensma (onasemnogene abeparvovec-xioi) or Itvisma (onasemnogene abeparvovec-brve) for any other indication or use is considered not medically necessary by the Plan, as it is deemed to be experimental, investigational, unproven, or not medically necessary. Non-covered indications include, but are not limited to, the following:

- For Zolgensma (onasemnogene abeparvovec-xioi) and Itvisma (onasemnogene abeparvovec-brve):
    - Previous administration of Zolgensma (onasemnogene abeparvovec-xioi) or Itvisma (onasemnogene abeparvovec-brve) precludes a new request for either gene therapy.
        - Rationale: Safety and effectiveness of treatment experienced gene-therapy individuals have not been evaluated.
    - Treatment in members with 4 or more copies of the SMN2 gene.
        - Rationale: These individuals are more likely to develop milder forms of SMA and the risk-benefit profile of treatment is less clear. Pivotal trials allowed for up to 3 copies of SMN2 (range of 1-3 copies).
    - Treatment in members with anti-AAV9 antibody titers >1:50.
        - Rationale: Higher antibody levels may affect treatment efficacy and safety. Retesting, however, may be performed if the anti-AAV9 antibody titers are reported as >1:50.
    - Repeat administration of Zolgensma or Itvisma.
        - Rationale: Safety and effectiveness of repeat administration have not been evaluated.
- For Zolgensma (onasemnogene abeparvovec-xioi):
    - Advanced SMA, including but not limited to:
        - Complete paralysis of limbs.
        - Permanent ventilator dependence (defined as ≥16 hours of respiratory assistance per day continuously for ≥14 days in the absence of an acute reversible illness or perioperative state).
        - Rationale: permanent ventilator dependence was a defined outcome measure of a pivotal trail, and Zolgensma (onasemnogene abeparvovec-xioi) was not studied in this population. Paralysis and permanent ventilator dependence are considered an advanced form of SMA, and Zolgensma (onasemnogene abeparvovec-xioi) has not been studied in this population.
    - Treatment in premature infants before reaching full-term gestational age.
        - Rationale: Concomitant use of corticosteroids may adversely affect neurological development. It is recommended to delay treatment with Zolgensma (onasemnogene abeparvovec-xioi) or until the corresponding full-term gestational age is reached.

Applicable Billing Codes

Table 1	
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
96450	Chemotherapy administration, into central nervous system (eg, intrathecal), requiring and including spinal puncture
J3399	Zolgensma Injection, onasemnogene abeparvovec-xioi, per treatment, up to 5x10^15 vector genomes
C9309	Itvisma Injection, onasemnogene abeprarvovex-brve, per treatment

Table 2	
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
G12.25	Progressive Spinal Muscle Atrophy
G12.8	Other spinal muscular atrophies and related syndromes
G12.9	Spinal muscular atrophy, unspecified

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