Zolgensma (abeparvovec-xioi)

BENEFIT APPLICATION

Benefit determinations are based on the applicable contract language in effect at the time the services were rendered. Exclusions, limitations or exceptions may apply. Benefits may vary based on contract, and individual member benefits must be verified. Wellmark determines medical necessity only if the benefit exists and no contract exclusions are applicable. This policy may not apply to FEP. Benefits are determined by the Federal Employee Program.

DESCRIPTION

The intent of the Zolgensma drug policy is to encourage appropriate selection of patients for therapy according to product labeling, clinical guidelines and clinical studies. Zolgensma (abeparvovec-xioi) is intended as a one-time gene replacement therapy designed to deliver a functional copy of the SMN1 gene to motor neuron cells of patients with SMA to allow for longterm, sustained SMN protein expression.

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indication

Zolgensma is indicated for the treatment of pediatric patients less than 2 years of age with spinal muscular atrophy (SMA) with bi-allelic mutations in the *survival motor neuron 1 (SMN1)* gene.

Limitations of use:

- The safety and effectiveness of repeat administrations of Zolgensma have not been evaluated.
- The use of Zolgensma in patients with advanced SMA (e.g., complete paralysis of limbs, permanent ventilator dependence) has not been evaluated.

POLICY

Required Documentation

Submission of the following information is necessary to initiate the prior authorization review Deletion or mutation at the SMN1 allele confirmed by genetic testing

- Genetic testing results demonstrating bi-allelic mutations in the *survival motor neuron 1 (SMN1)* gene
- Genetic testing results demonstrating the number of copies of the *survival motor neuron 2 (SMN2)* gene
- Enzyme-linked Immunosorbent Assay (ELISA) binding immunoassay results
- Medical records documenting patient does not have advanced SMA (e.g. respiratory status and need for respiratory support, limb paralysis, etc.)

Prescriber Specialties

This medication must be prescribed by or in consultation with a physician who specializes in treatment of spinal muscular atrophy.

Criteria for Initial Approval

A. Zolgensma (abeparvovec-xioi) may be considered **medically necessary** for the treatment of spinal muscular atrophy (SMA) in patients who meet the following criteria:

1. The member has a genetically confirmed diagnosis of SMA, with documentation of both of the following:
   a) There is genetic documentation of bi-allelic mutations in the *survival motor neuron 1 (SMN1)* gene (deletions or point mutations)
   b) There is documentation of no more than four copies of *survival motor neuron 2 (SMN2)*
2. The member is less than 2 years of age
3. The member does not have advanced SMA, including but not limited to any of the following:
   a) Complete paralysis of limbs
   b) Invasive ventilatory support (tracheostomy)
   c) Respiratory assistance for 16 or more hours per day (i.e. beyond use for naps and nighttime sleep), including non-invasive respiratory support, continuously for 14 or more days in the absence of acute reversible illness (excluding perioperative ventilation)
4. The member has an anti-adeno-associated virus 9 (AAV9) antibody titer less than or equal to 1:50 as determined by Enzyme-linked Immunosorbent Assay (ELISA) binding immunoassay
5. The medication is prescribed by or in consultation with a physician who specializes in treatment of spinal muscular atrophy and/or neuromuscular disorders
6. The member has not received Zolgensma previously
7. The member is not currently being treated with nusinersen (Spinraza) OR whom treatment with nusinersen will be discontinued prior to administration of Zolgensma (abeparvovec-xioi)
8. The member is not currently being treated with risdiplam (Evrysdi) OR whom treatment with risdiplam will be discontinued prior to administration of Zolgensma (abeparvovec-xioi)

Approval will be for one dose per lifetime with an authorization period of 14 days ending on the date of the anticipated administration from the provider or until member is 2 years of age, whichever is first.

B. Zolgensma (abeparvovec-xioi) is considered **investigational** for conditions not outlined in this policy, including but not limited to the following:

- Patients with advanced SMA (e.g., complete paralysis of limbs, permanent ventilator dependence)
- Repeat treatment or ante-partum use of abeparvovec-xioi (Zolgensma)
- Concomitant use of nusinersen (Spinraza) and abeparvovec-xioi (Zolgensma)
- Concomitant use of risdiplam (Evrysdi) and abeparvovec-xioi (Zolgensma)
- Symptomatic later-onset SMA older than 2 years of age
- SMA without chromosome 5q mutations or deletions
- SMA that is not attributed to the bi-allelic mutations of SMN1 gene

Zolgensma is considered **not medically necessary** for members who do not meet the criteria set forth above.

The Evio platform is a provider portal that is used to capture clinical outcome information for patients on select high-cost treatments, such as gene and cellular therapies. If a patient meets medical necessity as defined by this policy and is approved for treatment, the requesting physician must attest to providing clinical outcome information within the Evio provider portal at the requested cadence.

Dosage and Administration

Approvals may be subject to dosing limits in accordance with FDA-approved labeling, accepted compendia, and/or evidence-based practice guidelines. If an individual meets medically necessary criteria, dosing of abeparvovec-xioi treatment is covered according to the Food and Drug Administration (FDA) product information label. The FDA recommends that a single dose of 1.1 x 10^{14} vector genomes (vg) per kg of body weight should be administered.

Quantity Limits Apply
Zolgensma: 1 kit per lifetime

PROCEDURES AND BILLING CODES

To report provider services, use appropriate CPT* codes, Alpha Numeric (HCPCS level 2) codes, Revenue codes, and/or ICD Diagnostic Codes.

- J3399 - Injection, onasemnogene abeparvovec-xioi, per treatment, up to 5x10^15 vector genomes (effective 7/1/2020)

REFERENCES

- Zolgensma [package insert]. Bannockburn, IL. AveXis, Inc; February 2023.
- Institute for Clinical and Economic Review (ICER). Spinraza® and Zolgensma® for Spinal Muscular Atrophy: Effectiveness and Value. Final Evidence Report April 3, 2019 (Updated May 24, 2019; Confidential Data Unmasked November 2, 2020).
- Stevens D et al. Onasemnogene Abeparvovec-xioi: Gene Therapy for Spinal Muscular Atrophy. *Ann Pharmacother*. 2020. Available at: https://pubmed.ncbi.nlm.nih.gov/32204605/.
- Nagendran S et al. Impact of Age and Motor Function in a Phase 1/2A Study of Infants With SMA Type 1 Receiving Single-Dose Gene Replacement Therapy. *Pediatr Neurol*. 2019;98:39-45.

*Some content reprinted from CVSHealth

POLICY HISTORY

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