Zolgensma (onasemnogene abeparvovec-xioi) Injection

Last Review Date: June 11, 2020	Number: MG.MM.PH.194

Medical Guideline Disclaimer

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The medication listed in this policy is not covered by the pharmacy benefit. Zolgensma is covered by the medical benefit and must be obtained by Accredo, our preferred medical specialty provider.

Length of Authorization

Coverage will be provided: For no longer than 14 days from approval or until 2 years of age, whichever is first

Guideline

I. INITIAL APPROVAL CRITERIA

Zolgensma is proven and medically necessary for one treatment per lifetime for the treatment of spinal muscular atrophy (SMA) in patients who meet ALL of the following criteria:

- Submission of medical records (e.g., chart notes, laboratory values) confirming the following:
    - The mutation or deletion of genes in chromosome 5q resulting in one of the following:
        - Homozygous gene deletion or mutation of SMN1 gene (e.g., homozygous deletion of exon 7 at locus 5q13); or

        - Compound heterozygous mutation of SMN1 gene (e.g., deletion of SMN1 exon 7 [allele 1] and mutation of SMN1[allele 2]); AND

- One of the following:
    - Diagnosis of symptomatic SMA by a neurologist with expertise in the diagnosis of SMA; or
    - Both of the following:
        - Diagnosis of likely Type I or II SMA based on the results of SMA newborn screening; and
        - Submission of medical records (e.g., chart notes, laboratory values) confirming that patient has 3 copies or less of SMN2 gene; AND
- For use in a neonatal patient born prematurely, the full-term gestational age has been reached; AND
- One of the following:
    - Both of the following:
        - Patient is less than or equal to 6 months of age; and
        - Patient does not have advanced SMA at baseline (e.g., complete paralysis of limbs); OR
    - All of the following:
        - Patient is greater than 6 months of age, but less than 2 years of age; and
        - One of the following:
            - Both of the following:
                - Patient has previously received Spinraza (nusinersen) for the treatment of Type I, or likely Type I or II SMA before 6 months of age with positive clinical response; AND
                - Submission of medical records (e.g., chart notes, laboratory values) confirming patient does not have advanced SMA as defined by the fact that the patient has not shown evidence of clinical decline while receiving Spinraza therapy; OR
            - Both of the following:
                - Patient has previously received Spinraza (nusinersen) for the treatment of later-onset SMA before 2 years of age with positive clinical response; AND
                - Submission of medical records (e.g., chart notes, laboratory values) confirming patient does not have advanced SMA as

defined by the fact that the patient has not shown evidence of clinical decline while receiving Spinraza therapy; OR

- Patient has recently been diagnosed with symptomatic later-onset SMA within the previous 6 months

- Submission of medical records (e.g., chart notes, laboratory values) confirming patient does not have advanced SMA as defined by the fact that patient's most recent CHOP INTEND score is greater than or equal to 40; AND
- Patient is less than or equal to 13.5 kg; AND
- Dose to be administered does not exceed one kit of Zolgensma; AND

- Patient is not dependent on either of the following:
  - Invasive ventilation or tracheostomy
  - Use of non-invasive ventilation beyond use for naps and nighttime sleep; AND
- Zolgensma is prescribed by a neurologist with expertise in the treatment of SMA; AND
- Patient is not to receive routine concomitant SMN modifying therapy (e.g., Spinraza) (patient's medical record will be reviewed and any current authorizations for SMN modifying therapy will be terminated upon Zolgensma approval; patient access to subsequent SMN modifying therapy will be assessed according to respective coverage policy of concomitant agent); AND
- Physician attests that the patient will be assessed for the presence of anti-AAV9 antibodies and managed accordingly; AND
- Physician attests that the patient will not receive Zolgensma if the most recent pre-treatment anti-AAV9 antibody titer is above 1:50*; AND
- Physician attests that the patient, while under the care of the physician, will be assessed by one of the following exam scales during subsequent office visits for a period not to exceed 3 years*†
  - Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND) scale during subsequent office visits while the patient is 2 to 3 years of age or younger*†; or
  - Hammersmith Functional Motor Scale Expanded (HFMSE) during subsequent office visits while the patient is 2 to 3 years of age or older; AND
- Physician acknowledges that EmblemHealth and ConnectiCare may request documentation, not more frequently than biannually, of follow-up patient assessment(s) including, but not necessarily limited to, serial CHOP INTEND or HFMSE assessments while the patient is under the care of the physician*†; AND

- Patient will receive prophylactic prednisolone (or glucocorticoid equivalent) prior to and following receipt of Zolgensma within accordance of the United States Food and Drug Administration (FDA) approved Zolgensma labeling; AND
- Patient will receive Zolgensma intravenously within accordance of the FDA approved labeling, 1.1 x 1014 vector genomes (vg) per kg of body weight; AND
- Patient has never received Zolgensma treatment in their lifetime; AND
- Authorization will be for no longer than 14 days from approval or until 2 years of age, whichever is first.

II. RENEWAL CRITERIA

Safety and effectiveness of repeat administration of Zolgensma have not been evaluated.

Dosing/Administration

Spinal muscular atrophy, Bi-allelic survival motor neuron 1 (SMN1) gene mutations

The recommended dose of Zolgensma is 1.1 × 1014 vector genomes per kilogram (vg/kg) of body weight

Premedication, initiate systemic corticosteroids equivalent to oral prednisolone 1 mg/kg/day 1 day prior to infusion and continue for a total of 30 days.

Limitations/Exclusions

Zolgensma is not considered medically necessary for indications other than those listed above due to insufficient evidence of therapeutic value.

Other Exclusions:

1. Age older than 2 years of age.
2. Combination treatment of SMA with concomitant SMN modifying therapy (e.g. Spinraza) or past treatment with a SMN modifying therapy.
3. Patient has previously received a gene therapy for SMA.
4. Dose greater than one kit of Zolgensma.
5. Pre-symptomatic treatment for patients who are unlikely to develop Type 1 or Type 2 SMA
6. SMA without chromosome 5q mutations or deletions.
7. Safety and effectiveness of repeat administration of Zolgensma have not been evaluated

Applicable Procedure Codes

J3490	Unclassified drugs (when specified as Zolgensma)
J3399	Injection, onasemnogene abeparvovec-xioi, per treatment, up to 5x10^15 vector genomes (Zolgensma). Effective Date: 07/01/2020
C9399	Unclassified drugs or biologics
J3590	Unclassified biologics

Applicable NDC's

71894-0120-xx	Zolgensma 2.6-3.0 kg Intravenous Kit (2x8.3 mL)
71894-0121-xx	Zolgensma 3.1-3.5 kg Intravenous Kit (2x5.5mL & 1x8.3mL)
71894-0122-xx	Zolgensma 3.6-4.0 kg Intravenous Kit (1x5.5mL & 2x8.3mL)
71894-0123-xx	Zolgensma 4.1-4.5 kg Intravenous Kit (3x8.3 mL)
71894-0124-xx	Zolgensma 4.6-5.0 kg Intravenous Kit (2x5.5mL & 2x8.3mL)
71894-0125-xx	Zolgensma 5.1-5.5 kg Intravenous Kit (1x5.5mL & 3x8.3mL)
71894-0126-xx	Zolgensma 5.6-6.0 kg Intravenous Kit (4x8.3 mL)
71894-0127-xx	Zolgensma 6.1-6.5 kg Intravenous Kit (2x5.5mL & 3x8.3mL)
71894-0128-xx	Zolgensma 6.6-7.0 kg Intravenous Kit (1x5.5mL & 4x8.3mL)
71894-0129-xx	Zolgensma 7.1-7.5 kg Intravenous Kit (5x8.3 mL)
71894-0130-xx	Zolgensma 7.6-8.0 kg Intravenous Kit (2x5.5mL & 4x8.3mL)
71894-0131-xx	Zolgensma 8.1-8.5 kg Intravenous Kit (1x5.5mL & 5x8.3mL)
71894-0132-xx	Zolgensma 8.6-9.0 kg Intravenous Kit (6x8.3 mL)
71894-0133-xx	Zolgensma 9.1-9.5 kg Intravenous Kit (2x5.5mL & 5x8.3mL)
71894-0134-xx	Zolgensma 9.6-10.0 kg Intravenous Kit (1x5.5mL & 6x8.3mL)
71894-0135-xx	Zolgensma 10.1-10.5 kg Intravenous Kit (7x8.3 mL)
71894-0136-xx	Zolgensma 10.6-11.0 kg Intravenous Kit (2x5.5mL & 6x8.3mL)
71894-0137-xx	Zolgensma 11.1-11.5 kg Intravenous Kit (1x5.5mL & 7x8.3mL)
71894-0138-xx	Zolgensma 11.6-12.0 kg Intravenous Kit (8x8.3 mL)
71894-0139-xx	Zolgensma 12.1-12.5 kg Intravenous Kit (2x5.5mL & 7x8.3mL)
71894-0140-xx	Zolgensma 12.6-13.0 kg Intravenous Kit (1x5.5mL & 8x8.3mL)
71894-0141-xx	Zolgensma 13.1-13.5 kg Intravenous Kit (9x8.3 mL)

Applicable Diagnosis Codes

ICD-10	ICD-10 Description
G12.0	Infantile spinal muscular atrophy, type I [Werdnig-Hoffmann]
G12.1	Other inherited spinal muscular atrophy
G12.9	Spinal muscular atrophy, unspecified

Revision History:

06/11/2020	Added J-Code (J3399): Injection, onasemnogene abeparvovec-xioi, per treatment, up to 5x10^15 vector genomes (Zolgensma). Effective Date: 07/01/2020

References

1. Product Information: ZOLGENSMA(R) intravenous suspension, onasemnogene abeparvovec-xioi intravenous suspension. AveXis Inc (per manufacturer), Bannockburn, IL, 2019.