RX.PA.038.CCH ZOLGENSMA® (ONASEMNOGENE ABEPARVOVEC)

The purpose of this policy is to define the prior authorization process for Zolgensma® (onasemnogene abeparvovec).

Zolgensma® (onasemnogene abeparvovec) 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.

POLICY

It is the policy of the Health Plan to maintain a prior authorization process that promotes appropriate utilization of specific drugs with potential for misuse or limited indications. This process involves a review using Food and Drug Administration (FDA) criteria to make a determination of Medical Necessity, and approval by the Medical Policy Committee.

The drug, Zolgensma® (onasemnogene abeparvovec), is subject to the prior authorization process.

DEFINITIONS

Spinal Muscular Atrophy (SMA) – an autosomal recessive neuromuscular disease characterized by degeneration of the motor neurons in the anterior horn of the spinal cord, resulting in atrophy of the voluntary muscles of the limbs and trunk. Despite being a rare disease, SMA is a leading genetic cause of infant mortality and a major cause of childhood morbidity. It is attributed to deletions or mutations in the SMN1 gene (chromosome 5q13), causing insufficient expression of survival motor neuron (SMN) protein. The lack of SMN protein appears to result in dysfunction and eventual death of motor neurons. SMA can present clinically at any time from in utero to adulthood with gross motor function deficits, muscle weakness, and pulmonary disease due to neuromuscular weakness. Common complications include difficulty feeding, swallowing, failure to thrive, loss of ambulation, scoliosis, joint contracture, pulmonary disease, and death

Spinal Muscular Atrophy Types I, II, III – Type I manifests around or before the patient is 6 months of age. The presentation may include: hypotonia, unable to control head movement, unable to sit without assistance. Type II has an onset between 6 to 18 months. Patients are generally able to sit independently, the ability to walk is usually not achieved without assistance. Type III manifests after the patient is 18 months of age or older. Patients may be able to walk without assistance or lose the ability to walk.

PROCEDURE

Initial Authorization Criteria:

Must meet all the criteria listed below:

- Must be prescribed by a neurologist who specializes in the treatment of spinal muscular atrophy
- Must be prescribed at a dose within the manufacturer's dosing guidelines (based on diagnosis, weight, etc.) listed in the FDA approved labeling
- Must be less than 2 years of age
    - If born premature, must have reached corresponding full-term gestational age
- Must have a diagnosis of spinal muscular atrophy (SMA) with chart documentation of confirmatory genetic testing showing ONE of the following:
    - Homozygous gene deletion or mutation of the survival motor neuron 1 (SMN1) gene (e.g., homozygous deletion of exon 7 at locus 5q13)
    - Compound heterozygote gene mutation [e.g., deletion of SMN1 exon 7 (allele 1) and mutation of SMN1 (allele 2)]
- Must have documentation of ONE of the following:
    - Symptomatic SMA **OR**
    - Both of the following:
        - Diagnosis of likely Type I or II SMA based on the results of SMA newborn screening
        - Documentation confirming 3 copies or less of SMN2 gene
- Must not have advanced SMA as evidenced by any of the following:
    - Permanent ventilator dependence (defined as 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)
    - Complete paralysis of limbs
- Must NOT be dependent on either invasive ventilation or tracheostomy, OR non-invasive ventilation for at least 12 hours per day
- Must have the following baseline testing completed:
    - Platelet count
    - Troponin-1
    - Liver function tests (ALT, AST, bilirubin, prothrombin time)
    - Anti-AAV9 (anti- adeno-associated virus serotype 9) antibodies titer ≤ 1:50
- Must not have previously received treatment with Zolgensma
- Must not be receiving concomitant SMA treatment (i.e., Zolgensma, Evrysdi)
- Must plan to receive 30 days of systemic corticosteroids equivalent to oral prednisolone at 1 mg/kg beginning 1 day prior to treatment with Zolgensma

Limitations:

Length of Authorization (if above criteria met)	
Initial Authorization	1-time approval per lifetime
Reauthorization	N/A (only indicated for one-time use)
Quantity Limit	
Zolgensma	• Weight based dosing- 1.1 × 1014 vector genomes (vg) per kg • Max of 9 vials

If the established criteria are not met, the request is referred to a Medical Director for review, if required for the plan and level of request.

HCPCS Code(s)

HCPCS Code	Brand	Description
J3399	ZOLGENSMA	INJECTION, ONASEMNOGENE ABEPARVOVEC-XIOI, PER TREATMENT, UP TO 5x10^15

REFERENCES

1. Zolgensma [prescribing information]. Bannockburn, IL; AveXis, 2019.

RECORD RETENTION

Records Retention for Evolent Health documents, regardless of medium, are provided within the Evolent Health records retention policy and as indicated in CORP.028.E Records Retention Policy and Procedure.

REVIEW HISTORY

DESCRIPTION OF REVIEW / REVISION	DATE APPROVED
Initial Review	3/22