Maryland
DEPARTMENT OF HEALTH

Zolgensma (onasemnogene abeparvovec)

Zolgensma (onasemnogene abeparvovec-xioi) is an adeno-associated virus vector-based gene therapy 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.*

I. Criteria for Initial Approval

Zolgensma is considered medically necessary for one treatment per lifetime for the treatment of spinal muscular atrophy (SMA) in patients less than 2 years of age who meet **ALL** of the following criteria, confirmed with supporting documentation.

- Diagnosis of:
    - SMA Type I by a pediatric neurologist with expertise in the diagnosis of SMA; or
    - Diagnosis of likely Type I SMA based on the results of SMA newborn screening
- Genetic testing confirmation of bi-allelic deletion or point mutations in the survival motor neuron 1 (SMN1) gene.
- Prescribed by a pediatric neurologist with expertise in the treatment of SMA.
- SMA - associated symptom onset before 6 months of age.
- Member does not have advanced SMA, including but not limited to any of the following;
    - Complete paralysis of limbs; or
    - Invasive ventilation or tracheostomy;
    - 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)
- Prescriber attests that baseline evaluation has been complete and there are no contraindications, including all black box warnings on the package insert.
- The patient has not previously received gene replacement therapy for SMA.
- If the member is on Nusinersen (Spinraza), it will be discontinued prior to administration of Zolgensma.

- Prescriber attest that subsequent evaluation and monitoring will be done according to the FDA label.

II. Dosing/Administration

Zolgensma must be administered according to the current FDA labeling guidelines for dosage and timing. The recommended dosing is as follows:

- Administered intravenously at a dose of 1.1×10^{14} vector genomes (vg) per kg of body weight.
- Dose to be administered does not exceed 1 kit.

IIII. Length of Authorization For initial therapy

Zolgensma will be authorized for 3 months after initial approval or until 2 years of age, whichever is first. Authorization is for one administration per lifetime.

IV. Billing Code/Information

HCCPS Code: J3399 Injection, Onasemnogene abeparvovec-xioi, per treatment, up to $5x10^{15}$ vector genomes

Prior authorization of benefits is not the practice of medicine nor the substitute for the independent medical judgment of a treating medical provider. The materials provided are a component used to assist in making coverage decisions and administering benefits. Prior authorization does not constitute a contract or guarantee regarding member eligibility or payment. Prior authorization criteria are established based on a collaborative effort using input from the current medical literature and based on evidence available at the time.

Approved by MDH Clinical Criteria Committee:1/1/2020
Last Reviewed Date: 1/4/2021

Revision History:

1/4/2021- Effective 1/1/2021, MCOs are required to cover Zolgensma per program guidance. Prior clinical criteria statements referencing MCO Carve Out are no longer applicable and therefore removed.