Zolgensma® (onasemnogene abeparvovec-xioi)
J3399 1 unit = 1 Therapeutic Dose

MS Medicaid Program:	Prior Authorization	**Effective Date**	02/15/2024
Revision Number:	1.0	**Last Rev Date:**	02/15/2024
Reviewed By:	Telligen Pharmacy Director	**Next Rev Date:**	
Approved By:	MS DOM Pharmacy Director	**Approved Date:**	02/15/2024

Indications	
Medication:	onasemnogene abeparvovec-xioi
Brand Name	Zolgensma®
Indications:	ZOLGENSMA 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.

Prior Authorization (PA) Criteria

Patient Demographics

A. Age of patient is within the age range as recommended by the FDA label; **AND**
B. Full term gestational age has been reached if patient is a neonate born prematurely, **AND**
C. There are no contraindications or documented intolerance to corticosteroid therapy.

Diagnostic Criteria

A. Diagnosis of symptomatic SMA, **OR**
B. **Each** of the following:
   a. Patient has been diagnosed with SMA based on the results of newborn screening, **AND**
   b. Genetic testing confirming 4 or fewer copies of SMN2 gene, **AND**
   c. Genetic testing confirms the presence of ONE of the following:
      i. Homozygous deletions of SMN1gene (e.g. homozygous deletion of exon 7 at locus 5q13), **OR**
      ii. Homozygous mutation in the SMN1 gene (e.g. biallelic mutations of exon 7), **OR**
      iii. Compound heterozygous mutation in the SMN1 gene (e.g. deletion of SMN1 exon 7 (allele 1) and mutation of SMN1 (allele 2)]

Previous Treatment

If the patient is on nusinersen (Spinraza) or risdiplam (Evrysdi), it will be discontinued prior to administration of Zolgensma.

Current Status

Patient must not have advanced SMA, including but not limited to any of the following:

1. Complete paralysis of limbs **OR**
2. Invasive ventilator support (tracheostomy) **OR**
3. Respiratory assistance for more than 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)

Diagnostic/Treatment Teams

Diagnosed and prescribed by a pediatric neuromuscular specialist or a neurologist with experience in the diagnosis and management of SMA.

Concomitant Therapy

Patient will receive prophylactic prednisolone (or glucocorticoid equivalent) prior to and following receipt of Zolgensma within accordance of the FDA approved Zolgensma labeling.

Baseline Information

A. Documentation of baseline laboratory tests demonstrating Anti-AAV9 titers < 1:50 as determined by ELISA binding immunoassay, (if Anti-AAV titers > 1:50, retesting may be performed provided age requirement at time of dosing is still met) **AND**

B. Documentation of baseline liver function test, platelet counts, and troponin-I **AND**

C. Patient must not have advanced SMA, including but not limited to any of the following:

1. Complete paralysis of limbs **OR**
2. Invasive ventilator support (tracheostomy) **OR**
3. Respiratory assistance for more than 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).

Other Commitments

A. Physician attests that liver function tests, platelet counts, and troponin-I laboratory monitoring as recommended in the package insert or in compliance with current standards will be performed to assess safety; **AND**

B. Zolgensma is dosed in accordance of the FDA approved Zolgensma labeling* **AND**

C. Zolgensma will be administered intravenously over 60 minutes.

NOTES

- Single, one-time infusion per lifetime.
- Dose to be administered does not exceed one kit of Zolgensma.
- Infusion may be performed up to 14 days from approval or until 2 years of age, whichever is first, from time of authorization.

*For children less than 2 years of age who are otherwise eligible for Zolgensma therapy and who are over 21 kg, special arrangements for drug delivery will apply and the manufacturer must be contacted.