ALASKA MEDICAID
Prior Authorization Criteria

Zolgensma®
(onasemnogene abeparvovec-xioi)

FDA INDICATIONS AND USAGE[1]

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.

Limitations of Use

- The safety and effectiveness of repeat administration 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.

APPROVAL CRITERIA[1,2]

1. Patient meets FDA approved age range **AND**;
2. Prescribed by or in consultation with a pediatric neurologist **AND**;
3. Patient has a diagnosis of spinal muscular atrophy (SMA) **AND;**
4. Patient has one of the following confirmed by genetic testing
   (1) Homozygous gene deletion or mutation of SMN1 gene **OR;**
   (2) Compound heterozygous mutation of the SMN1 gene **AND;**
5. Patient has an anti-AAV9 antibody titer ≤ 1:50 **AND;**
6. Patient will not receive concomitant treatment with other SMA modifying therapy (e.g. nusinersen, risdiplam) **AND;**
7. Baseline laboratory tests (including LFT, platelet count, and Troponin-I) conducted within 30 days of request < 2 times the upper limit of normal (ULN).

DENIAL CRITERIA [1,2]

1. Failure to meet approval criteria **OR**;
2. Patient has been previously treated with Zolgensma **OR;**
3. Patient SMA is at an advanced stage (e.g. complete paralysis of limbs, permanent ventilator dependence, tracheostomy) **OR;**
4. Patient currently has an active, unresolved infection

CAUTIONS[1]

- If liver function abnormalities continue to persist ≥ 2 times ULN after the 30-day period of systemic corticosteroids, promptly consult a pediatric gastroenterologist or hepatologist.
- Cases of acute liver failure have been reported. Patients with preexisting hepatic impairment may be at elevated risk.

DURATION OF APPROVAL

- Initial Approval: 3 months
    - No reauthorization will be approved.

QUANTITY LIMIT

- One infusion per lifetime.
    - Total dose not to exceed 1.1 $x10^{14}$ vector genomes (vg) per kg.
- HCPCS: J3590

REFERENCES / FOOTNOTES:

1. Zolgensma [package insert]. Bannockburn, IL; Novartis Gene Therapies, Inc.; February 2023
2. Institute for Clinical and Economic Review (ICER). Final Evidence Report- Spinraza and Zolgensma for spinal muscular atrophy: effectiveness and value. https://icer.org/wp-content/uploads/2020/10/ICER_SMA_Final_Evidence_Report_110220.pdf. Accessed August 8 2023.