Reference number(s)
3093-A

SPECIALTY GUIDELINE MANAGEMENT

ZOLGENSMA (abeparvovec-xioi)

POLICY

I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indication
Zolgensma 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 (SMN1)* gene.

Limitations of use:
- The safety and effectiveness of repeat administrations 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.

All other indications are considered experimental/investigational and are not a covered benefit.

II. DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review:
1. Genetic testing results demonstrating bi-allelic mutations in the *survival motor neuron 1* (*SMN1)* gene
2. Genetic testing results demonstrating the number of copies of the *survival motor neuron 2* (*SMN2)* gene

III. CRITERIA FOR INITIAL APPROVAL

Spinal muscular atrophy
Authorization of one dose total may be granted for treatment of spinal muscular atrophy when all of the following criteria are met:
1. Member has a genetically confirmed diagnosis of SMA type 1, with documentation of both of the following:
   a. There is genetic documentation of bi-allelic mutations in the *survival motor neuron 1 (SMN1)* gene (deletions or point mutations)
   b. There is documentation of at least two copies of *survival motor neuron 2 (SMN2)*
2. Member does not have advanced SMA, including but not limited to any of the following:
   a. Complete paralysis of limbs
   b. Invasive ventilatory support (tracheostomy)
   c. 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)
3. The member has an anti-adeno-associated virus 9 (AAV9) antibody titer less than or equal to 1:50 as determined by Enzyme-linked Immunosorbent Assay (ELISA) binding immunoassay

4. The medication is prescribed by or in consultation with a physician who specializes in treatment of spinal muscular atrophy
5. If the member is on nusinersen (Spinraza), it will be discontinued prior to administration of the requested drug
6. The member has not received Zolgensma previously
7. The member is less than 9 months of age at time of administration

IV. REFERENCES

1. Zolgensma [package insert]. Bannockburn, IL. AveXis, Inc; May 2019.