Reference number(s)
3093-A

SPECIALTY GUIDELINE MANAGEMENT

ZOLGENSMA (onasemnogene 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 1 (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 not medically necessary.

II. DOCUMENTATION

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

III. PRESCRIBER SPECIALTIES

This medication must be prescribed by or in consultation with a physician who specializes in treatment of spinal muscular atrophy.

IV. 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:
A. Member has a genetically confirmed diagnosis of SMA, with documentation of bi-allelic mutations in the *survival motor neuron 1 (SMN1)* gene (deletions or point mutations).
B. Member is less than 2 years of age.
C. Member does not have advanced SMA, including but not limited to any of the following:
    1. Complete paralysis of limbs
    2. Invasive ventilatory support (tracheostomy)

3. 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)

D. 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.
E. If the member is on nusinersen (Spinraza) or risdiplam (Evrysdi), it will be discontinued prior to administration of the requested drug.
F. The member has not received Zolgensma previously.

V. REFERENCES

1. Zolgensma [package insert]. Bannockburn, IL. Novartis Gene Therapies, Inc; February 2023.