Physician Administered Drugs

ZOLGENSMA® (onasemnogene abeparvovec-xioi)

J3399 1 unit = 1 Therapeutic Dose

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.

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 (37 weeks) 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. Genetic testing confirms SMA with the presence of **ONE** of the following:

a. Homozygous deletions of SMN1 gene (e.g., homozygous deletion of exon 7 at locus 5q13)

OR

b. Homozygous mutation in the SMN1 SMN1 gene (e.g., biallelic mutations of exon 7)

OR

c. Compound heterozygous mutation in the SMN1 gene [e.g., deletion of SMN1 exon 7 (allele 1) and mutation of SMN1 (allele 2)]

AND

B. Genetic testing confirming 4 or fewer copies of SMN2 gene

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:

A. Complete paralysis of limbs

OR

B. Invasive ventilator support (tracheostomy)

OR

C. 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 $\leq$ 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

Documentation of baseline liver assessments (e.g., alanine aminotransferase, total bilirubin, gamma-glutamyl transferase or glutamate dehydrogenase), platelet counts, and troponin-I less than the upper limit of normal

Other commitments

A. Physician attests that liver assessments (e.g., alanine aminotransferase, total bilirubin, gamma-glutamyl transferase or glutamate dehydrogenase), platelet counts, and troponin-I ; as recommended in the package insert or in compliance with current standards will be performed to assess safety

AND

B. Zolgensma will be 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.