

## Zolgensma® (onasemnogene abeparvovec-xioi)

When requesting Zolgensma® (onasemnogene abeparvovec-xioi), the individual requiring treatment must be diagnosed with an FDA-approved indication and meet the specific coverage guidelines and applicable safety criteria for the covered indication.

### FDA-Approved Indication

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.

### Coverage Guidelines

#### Treatment of spinal muscular atrophy (SMA)

An individual must meet **all** of the following criteria for approval:

- Has had a genetic test confirming the diagnosis of SMA with bi-allelic pathogenic variants in the *survival motor neuron 1 (SMN1)* gene (e.g., homozygous deletion, compound heterozygous mutation, or a variety of other rare mutations) [documentation required];
- Is less than 2 years of age;
- If the individual is a premature neonate, full term gestational age of 39 weeks and 0 days has been met;
- Meets one of the following:
  - Has three or less *survival motor neuron 2 (SMN2)* gene copies [documentation required]; OR
  - Has four *survival motor neuron 2 (SMN2)* gene copies determined by a quantitative assay capable of distinguishing between four *SMN2* gene copies and five or greater *SMN2* gene copies [documentation required];
- Has baseline anti-AAV9 antibody titers of  $\leq 1:50$  [documentation required];
- Has not received Zolgensma in the past;
- For a patient currently receiving or who has received prior treatment with Spinraza (nusinersen injection for intrathecal use), further therapy with Spinraza will be discontinued;
- For a patient currently receiving or who has received prior treatment with Evrysdi (risdiplam oral solution), further therapy with Evrysdi will be discontinued;
- Will be receiving systemic corticosteroids equivalent to oral prednisolone at a dose of 1 mg/kg per day starting 1 day prior to Zolgensma infusion and for a total of 30 days;
- Has undergone a renal function assessment within the last 30 days and has a creatinine level less than 1.0 mg/dL [documentation required];

- Has undergone a liver function assessment within the last 30 days and meets ALL of the following conditions [documentation required]:
  - Alanine aminotransferase levels are  $\leq 2x$  the upper limit of normal (ULN);
  - Aspartate aminotransferase levels are  $\leq 2x$  the ULN;
  - Total bilirubin levels are  $\leq 2x$  the ULN;
  - Prothrombin time results are  $\leq 2x$  the ULN
- Has obtained a complete blood count within the last 30 days and meets BOTH of the following conditions [documentation required]:
  - White blood cell count is  $\leq 20,000$  cells/mm<sup>3</sup>;
  - Hemoglobin levels are between 8 g/dL and 18 g/dL;
- Zolgensma is prescribed by or in consultation with a physician who specializes in the management of spinal muscular atrophy and/or neuromuscular disorders.

**Approval duration:** One dose (lifetime)

## Dosing Recommendation

Zolgensma is for single-dose intravenous infusion only.

The recommended dose is  $1.1 \times 10^{14}$  vector genomes per kilogram (vg/kg) of body weight.

## References

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