

Zolgensma® (onasemnogene abeparvovec-xioi)

When requesting Zolgensma® (onasemnogene abeparvovec-xioi), the individual requiring treatment must be diagnosed with the following 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;
Note: Full-term gestational age can be defined as the postmenstrual age (gestational age plus chronological age) being ≥ 39 weeks and 0 days.
- 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 or Itivisma (onasemnogene abeparvovec-brve intrathecal injection) 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 and tablets), further therapy with Evrysdi will be discontinued;

- Has undergone a renal function assessment within the past 30 days and has a creatinine level less than 1.0 mg/dL [documentation required];
- Has undergone liver function testing within the past 30 days and meets ALL of the following conditions [documentation required]:
 - o Alanine aminotransferase levels are $\leq 2x$ the upper limit of normal (ULN);
 - o Aspartate aminotransferase levels are $\leq 2x$ the ULN;
 - o Total bilirubin levels are $\leq 2x$ the ULN;
 - o Prothrombin time results are $\leq 2x$ the ULN
- Has obtained a complete blood count within the past 30 days and meets BOTH of the following conditions [documentation required]:
 - o White blood cell count is $\leq 20,000$ cells/mm³;
 - o Hemoglobin level is within the normal reference range;
- 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 (per lifetime)

Dosing Recommendation

Zolgensma is for single-dose intravenous infusion only.

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

References

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8. Evrysdi® oral solution [prescribing information]. South San Francisco, CA: Genentech/Roche; February 2026.
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17. Glascock J, Sampson J, Connolly AM, et al. Revised recommendations for the treatment of infants diagnosed with spinal muscular atrophy via newborn screening who have 4 copies of SMN2. *J Neuromuscul Dis*. 2020;7(2):97-100.
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