

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 mutations in the *survival motor neuron 1 (SMN1)* gene reported as at least one of the following: homozygous deletion, homozygous mutations, or compound heterozygous mutation;
- 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;
- Has three or less *survival motor neuron 2 (SMN2)* gene copies;
- Has baseline anti-AAV9 antibody titers of $\leq 1:50$;
- Has not received Zolgensma in the past;
- For patients who have received prior treatment with Spinraza (nusinersen injection for intrathecal use), further therapy with Spinraza will be discontinued;
- For patients who have 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;
- The following laboratory tests will be evaluated prior to Zolgensma administration: baseline liver function tests, platelet counts, and Troponin-I levels; AND
- 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 1.1×10^{14} vector genomes per kilogram (vg/kg) of body weight.

References

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7. Spinraza® injection for intrathecal use [prescribing information]. Cambridge, MA: Biogen; June 2020.
8. Evrysdi™ oral solution [prescribing information]. South San Francisco, CA; Genentech (a Member of the Roche Group); August 2020.
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11. Al-Zaidy SA, Pickard AS, Kotha K, et al. Health outcomes in spinal muscular atrophy type 1 following AVXS-101 gene replacement therapy. *Pediatr Pulmonol.* 2019;54:179-185.
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14. 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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