

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:

- Had a genetic test done 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
- 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
- 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 following laboratory assessments completed or will be completed prior to Zolgensma administration: baseline liver function tests, platelet counts, and Troponin-I levels
- 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

1. Zolgensma® suspension for intravenous infusion [prescribing information]. Bannockburn, IL: AveXis; May 2019.
2. Nash LA, Burns JK, Chardon JW, et al. Spinal muscular atrophy: more than a disease of motor neurons? *Curr Mol Med*. 2016;16(9):779-792.
3. Arnold ES, Fischbeck KH. Spinal muscular atrophy. *Handb Clin Neurol*. 2018;148:591-601.
4. Mendell JR, Al-Zaidy S, Shell R, et al. Single-dose gene replacement therapy for spinal muscular atrophy. *N Engl J Med*. 2017;377(18):1713-1722.
5. Glascock J, Sampson J, Haidet-Phillips A, et al. Treatment algorithm for infants diagnosed with spinal muscular atrophy through newborn screening. *J Neuromuscular Dis*. 2018;5:145-158.
6. Nash LA, Burns JK, Chardon JW, et al. Spinal muscular atrophy: more than a disease of motor neurons. *Curr Mol Med*. 2016;16(9):779-792.