

Itvisma® (onasemnogene abeparvovec-brve)

When requesting Itvisma® (onasemnogene abeparvovec-brve), 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

Itvisma is an adeno-associated virus vector-based gene therapy indicated for the treatment of spinal muscular atrophy (SMA) in adult and pediatric patients 2 years of age and older with confirmed mutation 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 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 greater than 2 years of age to 18 years of age;
- Meets one of the following:
 - Has two or three *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 Itvisma or Zolgensma in the past (confirmed by claim history or prescriber attestation);
- 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 *[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 past 30 days and meets BOTH of the following [documentation required]:
 - White blood cell count is $\leq 20,000$ cells/mm³;
 - Hemoglobin level is within normal reference range;
- Does not have hepatitis B and hepatitis C;
- Is not HIV positive;
- Itvisma 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 Recommendations

Itvisma is for single-dose intrathecal injection only.

The recommended dose is 1.2×10^{14} vector genomes.

References

1. Itvisma® intrathecal injection [prescribing information]. Bannockburn, IL: Novartis; November 2025.
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3. Yeo CJJ, Tizzano EF, Darras BT. Challenges and opportunities in spinal muscular atrophy therapeutics. *Lancet Neurol*. 2024;23:205-218.
4. Ramdas S, Oskoui M, Servais L. Treatment options in spinal muscular atrophy: a pragmatic approach for clinicians. *Drugs*. 2024;84:747-762.
5. Prior TW, Leach ME, Finanger E. Spinal Muscular Atrophy. 2000 Feb 24 [Updated 2024 September 19]. In: Adam MP, Feldman J, Mirzaa GM, et al, editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2024. Available at: https://www.ncbi.nlm.nih.gov/books/NBK1352/pdf/Bookshelf_NBK1352.pdf. Accessed on January 14, 2026.
6. Proud CM, Vu DC, Wilmschurst JM, et al, for the STEER study group. Intrathecal onasemnogene abeparvovec in treatment-naïve patients with spinal muscular atrophy: a phase 3, randomized, controlled trial. *Nat Med*. 2025 Dec 8. [Online ahead of print].
7. Glascock J, Sampson J, Haidet-Phillips A, et al. Treatment algorithm for infants diagnosed with spinal muscular atrophy through newborn screening. *J Neuromuscul Dis*. 2018;5:145-158.
8. 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*.