

Reference number(s)
7318-A

# Specialty Guideline Management

## Itvisma

### Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

Brand Name	Generic Name
Itvisma	onasemnogene abeparvovec-brve

### Indications

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

#### FDA-approved Indications<sup>1</sup>

Itvisma is indicated for the treatment of spinal muscular atrophy (SMA) in adults and pediatric patients 2 years of age and older with confirmed mutation in survival motor neuron 1 (SMN1) gene.

All other indications are considered experimental/investigational and not medically necessary.

### Documentation

Submission of the following information is necessary to initiate the prior authorization review:

- Genetic testing results demonstrating bi-allelic pathogenic variants in the survival motor neuron 1 (SMN1) gene.
- Laboratory assay (e.g., quantitative PCR or MLPA) identifying copies of SMN2 gene.

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- Medical records (e.g., chart notes and/or laboratory reports) documenting baseline liver function, platelet count, troponin I level, creatinine level, neurologic evaluation, and Hammersmith Functional Motor Scale-Expanded (HFMSE) assessment.

## Prescriber Specialties

This medication must be prescribed by or in consultation with a physician who specializes in treatment of spinal muscular atrophy.

## Coverage Criteria

### Spinal Muscular Atrophy<sup>1-5</sup>

Authorization of one dose total may be granted for treatment of spinal muscular atrophy (SMA) when all of the following criteria are met:

- Member has a genetically confirmed diagnosis of SMA, with documentation of bi-allelic pathogenic variants in the survival motor neuron 1 (SMN1) gene (deletions or point mutations).
- Member has 3 or less copies of SMN2 gene.
- Member's onset of clinical signs and symptoms of disease occurred at 6 months of age or older.
- Member is 2 to less than 18 years of age at the time of treatment administration.
- Member does not require invasive ventilation, awake noninvasive ventilation for greater than 6 hours during a 24-hour period, noninvasive ventilation for greater than 12 hours during a 24-hour period, or require tracheostomy.
- Member does not have contraindication(s) to lumbar puncture procedure (e.g., increased intracranial pressure, any impediment to cerebrospinal fluid access, administration of any intrathecal therapy).
- Member has an anti-adenovirus 9 (AAV9) antibody titer less than or equal to 1:50 as determined by Enzyme-linked Immunosorbent Assay (ELISA) binding immunoassay.
- Member does not have an active infectious process (e.g. viral, bacterial, or febrile illness) prior to treatment.
- Member does not have a serious concomitant illness (e.g., severe liver or kidney disease, symptomatic cardiomyopathy).
- Member does not have a history of allergy or hypersensitivity to treatment regimen (e.g., glucocorticoids) or its excipients.
- Liver function, platelet count, troponin I level, creatinine level, neurologic evaluation, and Hammersmith Functional Motor Scale-Expanded (HFMSE) assessment have been assessed at baseline and will be monitored after Itvisma administration as clinically appropriate.
- Member's vaccination status will be up to date prior to Itvisma administration.

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- If the member is on nusinersen (Spinraza) or risdiplam (Evrysdi), it will be discontinued prior to administration of the requested drug.
- Member has not received Itvisma, Zolgensma, or other gene therapy previously.

## References

1. Itvisma [package insert]. Bannockburn, IL. Novartis Gene Therapies, Inc; November 2025.
2. ClinicalTrials.gov. Efficacy and Safety of Intrathecal OAV101 (AVXS-101) in Pediatric Patients with Type 2 Spinal Muscular Atrophy (SMA) (STEER). Identifier NCT05089656. Updated July 4, 2025. Accessed November 26, 2025.
3. Prior TW, Leach ME, Finanger EL. Spinal Muscular Atrophy. 2000 Feb 24 [Updated 2024 Sep 19]. In: Adam MP, Bick S, Mirzaa GM, et al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2026.
4. Proud CM, Vu DC, Wilmshurst JM, et al. Intrathecal onasemnogene abeparvovec in treatment-naive patients with spinal muscular atrophy: a phase 3, randomized controlled trial. *Nature Medicine*. 2025. doi:10.1038/s41591-025-04103-w.
5. Supplementary Material for: Intrathecal onasemnogene abeparvovec in treatment-naive patients with spinal muscular atrophy: A phase 3, randomized controlled trial. *Nature Medicine*. Published online 2025. doi:10.1038/s41591-025-04103-w.