Onasemnogene Abeparvovec-brve (Itvisma®)

IMPORTANT REMINDER

We develop Medical Policies to provide guidance to Members and Providers. This Medical Policy relates only to the services or supplies described in it. The existence of a Medical Policy is not an authorization, certification, explanation of benefits or a contract for the service (or supply) that is referenced in the Medical Policy. For a determination of the benefits that a Member is entitled to receive under his or her health plan, the Member's health plan must be reviewed. If there is a conflict between the medical policy and a health plan or government program (e.g., TennCare), the express terms of the health plan or government program will govern.

POLICY

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
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 number of SMN2 gene copies.
- 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 SPECIALITIES

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

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 fewer copies of the 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-adeno-associated virus 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.
- The dose of the requested medication will not exceed 1.2×10^{14} vector genomes (vg).
- 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.

APPLICABLE TENNESSEE STATE MANDATE REQUIREMENTS

BlueCross BlueShield of Tennessee's Medical Policy complies with Tennessee Code Annotated Section 56-7-2352 regarding coverage of off-label indications of Food and Drug Administration (FDA) approved drugs when the off-label use is recognized in one of the statutorily recognized standard reference compendia or in the published peer-reviewed medical literature.

ADDITIONAL INFORMATION

For appropriate chemotherapy regimens, dosage information, contraindications, precautions, warnings, and monitoring information, please refer to one of the standard reference compendia (e.g., the NCCN Clinical Practice Guidelines in Oncology (NCCN Guidelines®) published by the National Comprehensive Cancer Network®, Drugdex Evaluations of Micromedex Solutions at Truven Health, or The American Hospital Formulary Service Drug Information).

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 March 16, 2026.
3. Prior TW, Leach ME, Finanger EL. Spinal Muscular Atrophy. 2000 Feb 24 [Updated 2026 Feb 12]. In: Adam MP, Bick S, Mirzaa GM, et al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2026.

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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.

EFFECTIVE DATE 1/30/2027

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