Harvard Pilgrim Health Care | TUFTS Health Plan

Medical Necessity Guidelines

Medical Benefit Drugs

Itvisma (onasemnogene abeparvovec-brve)

Effective: July 1, 2026

Prior Authorization Required If REQUIRED, submit supporting clinical documentation pertinent to service request.	Yes ☒ No ☐

Applies to:

Commercial Products

☒ Harvard Pilgrim Health Care Commercial products; Fax 617-673-0988
☒ Tufts Health Plan Commercial products; Fax 617-673-0988

Public Plans Products

☒ Tufts Health Direct – A Massachusetts Qualified Health Plan (QHP) (a commercial product); Fax 617-673-0988
☐ Tufts Health Together – MassHealth Accountable Care Partnership Plans; Fax 617-673-0939
☒ Tufts Health RITogether – A Rhode Island Medicaid Plan; Fax 617-673-0939
☐ Tufts Health One Care-- A dual-eligible product; Fax 617-673-0956

Senior Products

☐ Tufts Health Plan Senior Care Options (SCO), (a dual-eligible product); Fax 617-673-0956
☐ Tufts Medicare Preferred HMO, (a Medicare Advantage product); Fax 617-673-0956
☐ Tufts Medicare Preferred PPO, (a Medicare Advantage product); Fax 617-673-0956

Note: While you may not be the provider responsible for obtaining prior authorization, as a condition of payment you will need to ensure that prior authorization has been obtained.

Overview

Spinal muscular atrophy (SMA) is a rare, inherited, autosomal recessive, neuromuscular disorder characterized by progressive degeneration of lower motor neurons in the spinal cord and brainstem, resulting in muscle weakness and atrophy. SMA is caused by biallelic (homozygous or compound heterozygous) pathogenic mutations or deletions in the survival motor neuron 1 (SMN1) gene. Loss of functional SMN1 leads to insufficient production of survival motor neuron (SMN) protein. Reduced SMN protein levels result in motor neuron death and progressive impairment of voluntary muscle function. SMA affects individuals across all racial and ethnic populations. The estimated incidence is approximately 1 in 6,000 to 1 in 10,000 live births. Disease severity is partly influenced by the number of copies of the SMN2 gene. The disease typically presents in infancy or early childhood, although later onset forms exist. Clinical severity varies and has historically been categorized by age of symptom onset and maximal motor milestone achieved. Common manifestations include hypotonia, symmetric proximal muscle weakness, loss or reduction of deep tendon reflexes, poor head and trunk control, feeding and swallowing difficulties, and progressive respiratory insufficiency.

Historically, treatment for SMA was limited to supportive and palliative care, including respiratory support, nutritional management, orthopedic interventions, and physical and occupational therapy. Advances in disease modifying therapies have substantially changed the clinical management of SMA.

Food and Drug Administration (FDA) Approved Indications:

- Itvisma (onasemnogene abeparvovec-brve) is an adeno-associated virus (AAV) 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 SMN1 gene.

For single-dose intrathecal injection only. The recommended dose is 1.2×10^{14} vector genomes (vg). Administer Itvisma as an intrathecal bolus injection over approximately 1 to 2 minutes. Postpone Itvisma in patients with infections until the infection has resolved and the patient is clinically stable. Prior to intrathecal injection, assess liver function by clinical examination and laboratory testing. Starting one day prior to Itvisma injection, administer systemic corticosteroids equivalent to oral prednisolone at 1 mg/kg of body weight per day for a total of 30 days. At the end of the 30-day period, check liver function by clinical examination and by laboratory testing (AST, ALT, total bilirubin). For patients with unremarkable findings, taper the corticosteroid dose gradually over

the next 28 days. If liver function abnormalities persist, continue systemic corticosteroids (equivalent to oral prednisolone at 1 mg/kg/day) until findings become unremarkable, and then taper the corticosteroid dose gradually over the next 28 days or longer if needed. Do not stop systemic corticosteroids abruptly. If at any time patients do not respond adequately to the equivalent of 1 mg/kg/day oral prednisolone, based on the patient's clinical course, prompt consultation with a gastroenterologist or hepatologist and adjustment to the recommended corticosteroid regimen may be considered.

Clinical Guideline Coverage Criteria

The Plan may cover a **one** dose intrathecal injection of Itvisma **per lifetime** (onasemnogene abeparvovec-brve) when **all** the following clinical criteria is met:

1. The Member is at least 2 years of age at time of treatment; **and**
2. The Member has SMA diagnosed by a neurologist with expertise in the treatment of SMA; **and**
3. Genetic testing/newborn screening confirms biallelic mutations (chromosome 5q related deletion or point mutations) in the survival motor neuron 1 (SMN1) gene and at least 2 but no more than 3 copies of the SMN2 gene; **and**
4. Pre-treatment testing confirms baseline anti-adeno-associated virus serotype 9 (anti-AAV9) antibody titers is ≤ 1:50 as determined by Enzyme-linked Immunosorbent Assay (ELISA) binding immunoassay; **and**
5. Documentation confirms that Member is not dependent on invasive ventilatory support or on non-invasive ventilator support (e.g., CPAP, BPAP) for greater than 16 hours/day; **and**
6. Documentation confirms that Member does not have advanced SMA (e.g., complete paralysis of limbs, permanent ventilator dependence); **and**
7. Documentation confirms Member has not received a previous infusion SMA gene therapy; **and**
8. If the Member is currently receiving an alternative SMA therapy (e.g., Spinraza (nusinersen), Evrysdi (risdiplam), the physician attests that treatment will be discontinued prior to infusion of Itvisma (defined as at least 4 months for Spinraza and at least 15 days for Evrysdi); **and**
9. The Member does not have active viral infection (e.g. respiratory, gastrointestinal, or chronic uncontrolled viral infection), including human immunodeficiency virus [HIV], hepatitis, or positive serology for hepatitis B or C, or Zika virus at the time of the infusion; **and**
10. Liver function, platelet count, troponin I, creatinine levels, and neurologic evaluation have been measured at baseline and will be monitored after Itvisma administration as clinically appropriate; **and**
11. The Member will receive prophylactic prednisolone (or glucocorticoid equivalent) prior to and following receipt of Itvisma in accordance with the FDA approved label directions.

Limitations

The plan will not cover Itvisma (onasemnogene abeparvovec-brve) under any of the following conditions:

- Itvisma for Members younger than 2 years of age
- Itvisma for the treatment of SMA type 0 or SMA type IV as the safety and efficacy for use in these conditions is unproven and considered investigational.
- Itvisma when genetic testing confirms more than 3 copies the SMN2 gene.
- Repeat treatment of Itvisma as the safety and effectiveness of repeat administration has not been evaluated and FDA approval has not been given. This includes previous intrathecal injection administered as part of a clinical trial or administered when Member was covered under a different health plan.
- Combination treatment of Itvisma with concomitant SMA modifying therapies (e.g. Spinraza, Evrysdi).

Codes

The following code(s) require prior authorization:

Table 1: CPT/HCPCS Codes

Codes	Description
J3405	Injection, onasemnogene abeparvovec-brve, per treatment

References:

1. Itvisma (onasemnogene abeparvovec-brve) [package insert]. Bannockburn, IL: Novartis. November 2025.
2. Clinicaltrials.gov. A Study of SmartFlow Magnetic Resonance (MR) Compatible Ventricular Cannula for Administering Eladocagene Exuparvovec to Pediatric Participants (NCT05089656). Available at: https://clinicaltrials.gov/study/NCT05089656. Accessed on December 30, 2025.

3. Proud CM, Vũ DC, et. al. STEER Study Group. Intrathecal onasemnogene abeparvovec in treatment-naive patients with spinal muscular atrophy: a phase 3, randomized controlled trial. Nat Med. 2026 Feb;32(2):481-487. doi: 10.1038/s41591-025-04103-w. Epub 2025 Dec 8. PMID: 41360993; PMCID: PMC12920101.
4. Proud CM, Vũ DC, Wilmshurst JM, et al. Supplementary information: Intrathecal onasemnogene abeparvovec in treatment-naive patients with spinal muscular atrophy: A phase 3, randomized controlled trial. Nature Medicine. 2025 Dec 8. Available at: https://static-content.springer.com/esm/art%3A10.1038%2Fs41591-025-04103-w/MediaObjects/41591_2025_4103_MOESM1_ESM.pdf
5. Kwon JM, Munell F, et. al. Intrathecal onasemnogene abeparvovec for treatment-experienced patients with spinal muscular atrophy: a phase 3b, open-label trial. Nat Med. 2026 Feb;32(2):488-493. doi: 10.1038/s41591-025-04119-2. Epub 2025 Dec 8. Erratum in: Nat Med. 2026 Feb;32(2):763. doi: 10.1038/s41591-026-04233-9. PMID: 41360995; PMCID: PMC12920129.
6. IPD Analytics New Drug Review; Itvisma (onasemnogene abeparvovec-brve). Available at https://www.ipdanalytics.com/ [subscription required].

Approval And Revision History

May 20, 2026: Reviewed by the Medical Policy Approval Committee (MPAC), effective July 1, 2026.

Background, Product and Disclaimer Information

Medical Necessity Guidelines are developed to determine coverage for benefits and are published to provide a better understanding of the basis upon which coverage decisions are made. We make coverage decisions using these guidelines, along with the Member's benefit document, and in coordination with the Member's physician(s) on a case-by-case basis considering the individual Member's health care needs.

Medical Necessity Guidelines are developed for selected therapeutic or diagnostic services found to be safe and proven effective in a limited, defined population of patients or clinical circumstances. They include concise clinical coverage criteria based on current literature review, consultation with practicing physicians in our service area who are medical experts in the particular field, FDA and other government agency policies, and standards adopted by national accreditation organizations. We revise and update Medical Necessity Guidelines annually, or more frequently if new evidence becomes available that suggests needed revisions.

For self-insured plans, coverage may vary depending on the terms of the benefit document. If a discrepancy exists between a Medical Necessity Guideline and a self-insured Member's benefit document, the provisions of the benefit document will govern. For Tufts Health Together (Medicaid), coverage may be available beyond these guidelines for pediatric members under age 21 under the Early and Periodic Screening, Diagnostic and Treatment (EPSDT) benefits of the plan in accordance with 130 CMR 450.140 and 130 CMR 447.000, and with prior authorization.

Treating providers are solely responsible for the medical advice and treatment of Members. The use of this guideline is not a guarantee of payment or a final prediction of how specific claim(s) will be adjudicated. Claims payment is subject to eligibility and benefits on the date of service, coordination of benefits, referral/authorization, utilization management guidelines when applicable, and adherence to plan policies, plan procedures, and claims editing logic.