Mass General Brigham
Health Plan

Medical Necessity Guidelines
Itvisma (onasemnogene abeparvovec-brve)

Policy Number: 112

Contents

Overview

Itvisma (onasemnogene abeparvovec-brve) is a one-time gene replacement therapy intended to treat spinal muscular atrophy (SMA) by delivering a functional copy of the SMN1 gene, thereby restoring SMN protein production to slow or halt neuromuscular degeneration.

Medicare Advantage

Prior Authorization Required	Yes ☒ No ☐

Mass General Brigham Health Plan uses guidance from the Centers for Medicare and Medicaid Services (CMS) for medical necessity determinations for its Medicare Advantage plan members. National Coverage Determinations (NCDs), Local Coverage Determinations (LCDs), Local Coverage Articles (LCAs), and documentation included in the Medicare manuals are the basis for medical necessity determinations. When there is no guidance from CMS for the requested service, Mass General Brigham Health Plan's medical policies are used for medical necessity determinations. **As of Mass General Brigham Health Plan's most recent policy review, Medicare had:**

- Medicare Benefit Policy Manual Chapter 15 – Covered Medical and Other Health Services

When CMS documentation references FDA labeling, Mass General Brigham Health Plan develops coverage criteria to clarify medical necessity of the requested services. Mass General Brigham Health Plan coverage criteria align with FDA labeling without contradicting existing determinations and enhance the clarity of medical necessity requirements, documentation requirements, and clinical indications.

FDA-Approved Indication

Itvisma (onasemnogene abeparvovec-brve) is an adeno-associated virus vector-based gene therapy for the treatment of SMA in patients with confirmed mutation in the SMN1 gene.

Criteria

1. Criteria for Initial Approval
   Authorization may be granted when all of the following criteria are met:
   a. The member has a genetically confirmed diagnosis of SMA with mutation in the SMN1 gene; and
   b. The member is at least 2 years of age; and
   c. The member's vaccination schedule will be adjusted to accommodate concomitant corticosteroid administration before and after Itvisma injection; and
   d. The member is clinically stable; and
   e. The member does not have any active infections.
2. Dosing and Administration
   a. The recommended dose of Itvisma is $1.2x10^{14}$ vector genomes administered over 1 to 2 minutes.

Mass General Brigham ACO

Prior Authorization Required	Yes ☒ No ☐

Prior authorization requests for Itvisma for Mass General Brigham ACO members should be submitted to the MassHealth Drug Utilization Review Program. Criteria for Itvisma are found in Table 76: Neuromuscular Agents – Duchenne Muscular Dystrophy and Spinal Muscular Atrophy.

One Care and Senior Care Options (SCO)

Prior Authorization Required	Yes ☒ No ☐

Mass General Brigham Health Plan uses guidance from CMS for medical necessity determinations for its One Care and SCO plan members. NCDs, LCDs, LCAs, and documentation included in the Medicare manuals are the basis for medical necessity determinations. When there is no guidance from CMS for the requested service, or the member does not meet all of the medical necessity criteria for the requested service, Mass General Brigham Health Plan uses medical necessity guidelines from MassHealth. **See Medicare Advantage criteria and exclusions above. If Medicare Advantage criteria are not met, then MassHealth criteria are applied.**

Commercial and Qualified Health Plans

Prior Authorization Required	Yes ☒ No ☐

Prior authorization for Itvisma for Commercial and Qualified Health Plan members is managed by Prime Therapeutics. See the Prime Therapeutics policy for Itvisma for more information.

Related Policies

- Medical Necessity Guidelines Zolgensma

Codes

The following codes are included below for informational purposes only; inclusion of a code does not constitute or imply coverage or reimbursement.

Authorized Code	Code Description
C9309	Injection, onasemnogene abeparvovec-brve, per treatment

Summary of Evidence

SMA is a rare, autosomal recessive neuromuscular disease caused by biallelic loss-of-function mutations in the SMN1 gene, resulting in insufficient survival motor neuron (SMN) protein production, progressive motor neuron degeneration, and muscle weakness (Bodamer, 2026). The severity of disease correlates inversely with SMN2 copy number, a paralogous gene that produces only a small fraction of functional SMN protein. Onasemnogene abeparvovec is an adeno-associated virus (AAV) vector-based gene therapy that works by delivering a functional copy of the SMN1 gene as a one-time treatment. The intravenous formulation (Zolgensma) was first studied in the pivotal phase 3 STR1VE trial, which enrolled 22 symptomatic infants under 6 months of age with SMA type 1. Of the 22 treated patients, 13 (59%) achieved functional independent sitting for at least 30 seconds at 18 months of age, compared with 0 of 23 patients in the untreated natural history cohort ($p<0.0001$), and 20 patients (91%) survived free from permanent ventilation at 14 months, versus only 6 (26%) in the historical comparator group ($p<0.0001$) (Day et al., 2021). While this intravenous formulation represented a major therapeutic advance, its weight-based dosing restricted use to patients under 2 years of age, leaving a substantial gap in care for older children, adolescents, and adults.

To address this unmet need, an intrathecal formulation of onasemnogene abeparvovec was developed, allowing a fixed dose to be delivered directly into the cerebrospinal fluid independent of patient weight. Early-phase evaluation of intrathecal delivery was conducted in the STRONG trial, a phase 1 ascending-dose study in sitting, nonambulatory patients aged 6 to under 60 months. At month 12, older patients (aged 24–60 months) treated with the medium dose demonstrated a statistically significant improvement in Hammersmith Functional Motor Scale–Expanded (HFMSE) score compared with the SMA historic control population ($P < 0.01$), and the therapy was considered safe with no deaths reported (Finkel et al., 2023). Building on this signal, the phase 3 STEER trial enrolled 126 treatment-naive patients aged 2 to less than 18 years with SMA type 2 who could sit but had never walked independently. Patients treated with intrathecal onasemnogene abeparvovec demonstrated a statistically significant improvement in HFMSE score versus sham (least squares mean difference of 1.88 points; 95% CI: 0.51–3.25; $P = 0.0074$), with an overall adverse event incidence that was similar between the treatment and sham groups (Proud et al., 2025). The FDA approved intrathecal onasemnogene abeparvovec (Itvisma, onasemnogene abeparvovec-brve) in November 2025, making it the first and only gene replacement therapy available for children aged 2 years and older, adolescents, and adults with SMA caused by a confirmed SMN1 mutation (IPD Analytics, 2025). The fixed-dose intrathecal formulation removes the weight-based dosing barrier that had historically restricted gene therapy access to the youngest patients.

Effective Dates

May 11, 2026: Effective date.

References

Bodamer O. Spinal muscular atrophy. UpToDate. Mar 16, 2026. Accessed on 3/20/2026 from https://www.uptodate.com/contents/spinal-muscular-atrophy.

Day JW, Finkel RS, Chiriboga CA, et al. Onasemnogene abeparvovec gene therapy for symptomatic infantile-onset spinal muscular atrophy in patients with two copies of SMN2 (STR1VE): an open-label, single-arm, multicentre, phase 3 trial. Lancet Neurol. 2021 Apr;20(4):284-293. doi: 10.1016/S1474-4422(21)00001-6. Epub 2021 Mar 17. PMID: 33743238.

Finkel RS, Darras BT, Mendell JR, et al. Intrathecal Onasemnogene Abeparvovec for Sitting, Nonambulatory Patients with Spinal Muscular Atrophy: Phase I Ascending-Dose Study (STRONG). J Neuromuscul Dis. 2023;10(3):389-404. doi: 10.3233/JND-221560. PMID: 36911944; PMCID: PMC10200150.

IPD Analytics. New drug review: Itvisma (onasemnogene abeparvovec-brve). Dec 23, 2025. Accessed on 3/20/2026 from https://secure.ipdanalytics.com/User/Handler/ViewReport.ashx?type=RP&file=s3%3a%2f%2fipdanalytics%2fReports%2fIPD+Analytics_RxInsights_New+Drug+Review_Itvisma_12+2025.pdf.

Itvisma [package insert]. Bannockburn, IL: Novartis Gene Therapies, Inc. Nov 2025.

Proud CM, Vũ DC, Wilmshurst JM, 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.