

Itvisma (onasemnogene abeparvovec-brve) PAM – 089

Iowa Medicaid Program	Prior Authorization	Effective Date	07/01/2026
Revision Number	1	Last Reviewed	10/16/2026
Reviewed By	Medicaid Medical Director	Next Review	04/16/2027
Approved By	Medicaid Clinical Advisory Committee	Approved Date	10/16/2026

Overview

Medication: ¹	onasemnogene abeparvovec-brve
Brand Name:	Itvisma®
Pharmacologic Category:	Adeno-associated virus (AAV) vector-based gene therapy
FDA-Approved Indication(s):	Treatment of spinal muscular atrophy (SMA) in adult and pediatric patients 2 years of age and older with confirmed mutation in <i>survival motor neuron 1 (SMN1)</i> gene
How Supplied:	Single-dose vial containing 1.2 x 10 ¹⁴ vector genomes (vg)
Dosage and Administration:	- Perform baseline testing for presence of anti-AAV9 antibodies - Should only be administered intrathecally using a lumbar puncture by healthcare professionals (e.g., interventional radiologist or neurologist) experienced in performing lumbar punctures - One-time dose of 1.2 x 10¹⁴ vector genomes (vg) - Patients previously treated with Zolgensma (onasemnogene abeparvovec-xioi) should not be treated with Itvisma
Benefit Category:	Medical

BOXED WARNING: SERIOUS LIVER INJURY

- Acute serious liver injury and elevated aminotransferases can occur with ITVISMMA.
- Patients with preexisting liver impairment may be at higher risk.
- Prior to intrathecal injection, assess liver function by clinical examination and laboratory testing. Administer systemic corticosteroid before and after ITVISMMA injection. Continue to monitor liver function for at least 3 months after injection, and at other times as clinically indicated.

Descriptive Narrative

Spinal muscular atrophy (SMA), an autosomal recessive genetic disorder, affects 1 in 10,000 live births and is the most common genetic cause of infant mortality. The majority of cases (over 95 percent) are caused by a homozygous deletion of *SMN1* gene, which encodes the survival motor neuron (SMN) protein.² Loss or mutation of both copies of the *SMN1* gene causes SMA. Critical deficiency of SMN protein results in degeneration of motor neurons in

the spinal cord, resulting in progressive muscle weakness and atrophy. Weakness and loss of response to stimuli in the lower extremities are most common, but in severe cases, the muscles that control the mouth, throat, and respiration may also be impacted.³

In combination with loss of *SMN1*, patients retain variable numbers of copies of a second similar gene, *SMN2*, which produce reduced levels of the survival motor neuron (SMN) protein that are insufficient for normal motor neuron function.⁴ A higher number of copies of *SMN2* often correlates to milder disease, but this correlation is a relative and not an absolute one.⁵

Classification of Spinal Muscular Atrophy

Spinal muscular atrophy has traditionally been classified into types 0-4 based on symptom severity and genotype, but with new therapies available (including nusinersen, risdiplam, and onasemnogene abeparvovec-xioi), phenotypes have become more diverse and classifications have evolved to focus on functional status or treatment response.

Classification of Spinal Muscular Atrophy ⁶					
SMA Type	Copies SMN2	Onset	Motor Milestones	Clinical Features	Natural History Prior to Disease-Modifying Therapy
0	1	Prenatal, at birth	Non-sitter, no head control	Generalized weakness, hypotonia, respiratory failure, poor feeding, contractures	Death within weeks of birth
1	1-2	0-6 months	Non-sitter	Proximal predominant weakness, respiratory insufficiency, poor feeding, tongue fasciculations	Death by age 2
2	3	6-18 months	Sits independently, never stands or ambulates	Proximal predominant weakness, tongue fasciculations, minipolymyoclonus, scoliosis	Most alive at 25 years
3	3-4	3a: 18 months – 3 years 3b: 3 – 30 years	Ambulates independently	Proximal, abnormal gait, lower extremity predominant weakness	Normal lifespan
4	4 or more	>30 yr	Ambulates independently	Maintain ability to ambulate	Normal lifespan

Guidelines

The most recent SMA best practice recommendations were published in 2018 shortly after the approval of the first SMN-enhancing treatment. According to a 2024 SMA Diagnosis Working Group, the availability of disease-modifying therapies for 5q SMA and implementation of SMA newborn screening (NBS) has led to urgency to update the SMA best practice recommendations for diagnosis and to reevaluate the current classification of SMA. In addition, the availability of disease-modifying therapies has opened the door to explore improved diagnosis of adult-onset SMA.⁷

Criteria

Prior authorization is required.

Itvisma® is considered medically necessary when **ALL** of the following are met:

1. Diagnosis of spinal muscular atrophy (SMA), with **ALL** of the following (a, b, and c):
 - a. Genetic documentation of bi-allelic mutation in *survival motor neuron 1 (SMN1)* gene; **AND**
 - b. Documentation of laboratory results confirming that the member has 4 or less copies of the *survival motor neuron 2 gene (SMN2)*; **AND**
 - c. Anti-adenovirus-associated viral vector, serotype 9 (AAV9) antibody titer is less than or equal to 1:50; **AND**
2. Member is ≥ 2 years of age and less than 18 years of age at the time of vector infusion; **AND**
3. Prescribed by, or in consultation with, a physician who specializes in the treatment of spinal muscular atrophy and/or neuromuscular disorders; **AND**
4. Both of the following regarding member's previous or current SMA therapy are met (a and b):
 - a. Member has not previously received Itvisma (onasemnogene abeparvovec-brve), Zolgensma (onasemnogene abeparvovec-xioi), or any other gene therapy for SMA; **AND**
 - b. If member is currently on other SMA therapy (e.g., Spinraza®, Evrysdi®, etc.), the current SMA therapy will be discontinued at or before the time of Itvisma® administration; **AND**
5. Member does not have findings of advanced SMA, including but not limited to:
 - a. complete paralysis of limbs; and/or
 - b. requires invasive ventilation; and/or
 - c. requires awake noninvasive ventilation for 6 or more hours during a 24-hour period; and/or
 - d. requires noninvasive ventilation for 12 or more hours during a 24-hour period; and/or
 - e. requires tracheostomy; **AND**
6. Member is able to sit independently (i.e., sit unassisted for 10 or more seconds) but has never had the ability to walk independently; **AND**
7. Dose does not exceed a single administration of 1.2×10^{14} vector genomes (vg) (may only be authorized for one administration per lifetime).

Approval Duration and Quantity Limits

	Initial Authorization	Subsequent Authorization(s)
Approval Duration	One course of treatment per lifetime, to be administered after member turns 2 years of age	Not applicable
Quantity Limits	One-time dose, not to exceed 1.2×10^{14} vector genomes (vg)	

Coding and Product Information

The following list(s) of codes and product information are provided for reference purposes only and may not be all inclusive. The inclusion of a code does not imply any right to reimbursement or guarantee claim payment, nor does the exclusion of a code imply that its association to the HCPCS code is inappropriate.

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

ICD-10	Description
G12.0	Infantile spinal muscular atrophy, type 1 (Werdnig-Hoffman)
G12.1	Other inherited spinal muscular atrophy (SMA) • Adult form SMA • Childhood form, type 2 SMA • Distal SMA • Juvenile form, type 3 SMA (Kugelberg-Welander) • Progressive bulbar palsy of childhood (Fazio-Londe) • Scapuloperoneal form SMA
G12.25	Progressive spinal muscular atrophy
G12.8	Other spinal muscular atrophies and related syndromes
G12.9	Spinal muscular atrophy, unspecified

NDC (Strength) (SDV: single-dose vial)	Labeler	Dosage	Pkg Size	Pkg Qty	Units/Pkg
71894-0200-01 (SFV, 1.2×10^{14} vg)	Novartis Gene Therapies, Inc. (71894)	per treatment	1	EA	1
71894-0200-02 (carton of one SDV)					

Compliance

1. Should conflict exist between the policy and applicable statute, the applicable statute shall supersede.
2. Federal and State law, as well as contract language, including definitions and specific contract provisions or exclusions, take precedence over medical policy and must be considered first in determining eligibility for coverage.
3. Medical technology is constantly evolving, and Iowa Medicaid reserves the right to review and update medical policy on an annual or as-needed basis.

Medical necessity guidelines have been developed for determining coverage for member benefits and are published to provide a better understanding of the

basis upon which coverage decisions are made. Medical necessity guidelines are developed for selected physician-administered medications found to be safe and proven to be effective in a limited, defined population or clinical circumstances. They include concise clinical coverage criteria based on current literature review, consultation with practicing physicians in the service area who are medical experts in the particular field, FDA and other government agency policies, and standards adopted by national accreditation organizations. Criteria are revised and updated annually, or more frequently if new evidence becomes available that suggests needed revisions.

References

- ¹ Itvisma® prescribing information (06/2026). Novartis Gene Therapies, Inc.: Bannockburn, IL. Available online: www.itvisma-hcp.com. Accessed July 2, 2026.
- ² Blaschek A, Kölbel H, et al. Newborn Screening for SMA - Can a Wait-and-See Strategy be Responsibly Justified in Patients With Four SMN2 Copies? *J Neuromuscul Dis.* 2022;9(5):597-605. PMID: 35848034.
- ³ Keinath MD, Prior DE, Prior TW. Spinal Muscle Atrophy: Mutations, Testing, and Clinical Relevance. *Appl Clin Genet.* 2021 Jan 25;14:11-25. PMID: 33531827.
- ⁴ Arnold WD, Kassar D, Kissel JT. Spinal muscular atrophy: diagnosis and management in a new therapeutic era. *Muscle Nerve.* 2015 Feb;51(2):157-67. Epub 2014 Dec 16. PMID: 25346245.
- ⁵ Calucho M, Bernal S, et al. Correlation between SMA type and SMN2 copy number revisited: An analysis of 625 unrelated Spanish patients and a compilation of 2834 reported cases. *Neuromuscul Disord.* 2018 Mar;28(3):208-215. Epub 2018 Jan 11. PMID: 29433793.
- ⁶ Keinath MD, Prior DE, Prior TW. Spinal Muscle Atrophy: Mutations, Testing, and Clinical Relevance. *Appl Clin Genet.* 2021 Jan 25;14:11-25. PMID: 33531827.
- ⁷ Schroth M, et al. Spinal Muscular Atrophy Update in Best Practices: Recommendations for Diagnosis Considerations. *Neurol Clin Pract.* 2024 Aug;14(4):e200310. Epub 2024 May 24. Erratum in: *Neurol Clin Pract.* 2025 Feb;15(1):e200386. PMID: 38915908; PMCID: PMC11195435.

Development of utilization management criteria may also involve research into other state Medicaid programs, other payer policies, consultation with experts and review by the Medicaid Clinical Advisory Committee (CAC). These sources may not be referenced individually unless they are specifically published and are otherwise applicable to the criteria at issue.

Criteria Change History

Change Date	Changed By	Description of Change	Version
[mm/dd/yyyy]	CAC		
Signature			

Change Date	Changed By	Description of Change	Version
[mm/dd/yyyy]	CAC		
Signature			

Change Date	Changed By	Description of Change	Version
10/16/2026	CAC	Criteria implementation.	1

Signature
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CAC = Medicaid Clinical Advisory Committee