BlueCross BlueShield
Federal Employee Program
Blue Cross Blue Shield Association
750 9th St NW, Suite 900
Washington, D.C. 20001
1-800-624-5060
Fax 1-877-378-4727

5.75.047

Section:	Prescription Drugs	**Effective Date:**	April 1, 2026
Subsection:	Neuromuscular Agents	**Original Policy Date:**	January 23, 2026
Subject:	Itvisma	**Page:**	1 of 5

Last Review Date: March 6, 2026

Itvisma

Description

Itvisma (onasemnogene abeparvovec-brve)

Background
Itvisma is a non-replicating recombinant adeno-associated virus (AAV) vector that utilizes AAV9 capsid to deliver a functional copy of human *survival motor neuron 1 gene* (*SMN1*). The transgene DNA persists largely in episomal form in the nucleus of transduced cells. Expression of the transgene is driven by a constitutive promoter (cytomegalovirus enhanced chicken β actin hybrid), resulting in continuous and sustained SMN expression. Spinal muscular atrophy (SMA) is caused by a bi-allelic mutation in the *SMN1* gene, which results in insufficient SMN protein expression. By providing an alternative source of SMN protein expression in motor neurons, it is expected to promote the survival and function of transduced motor neurons (1).

Regulatory Status
FDA approved indication: Itvisma 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 mutations in *SMN1* gene (1).

Itvisma has a boxed warning regarding serious liver injury. Acute serious liver injury and elevated aminotransferases can occur with Itvisma. Patients with pre-existing liver impairment or acute hepatic viral infection may be at higher risk. (1).

The recommended dose of Itvisma is 1.2×10^{14} vector genomes (vg) administered as an intrathecal bolus injection over approximately 1 to 2 minutes (1).

Administer systemic corticosteroid before and after Itvisma injection in order to mitigate potential aminotransferase elevations. Prior to Itvisma injection, liver function of all patients should be assessed by clinical examination and laboratory testing. Continue to monitor liver function for at least 3 months after Itvisma administration, and at other times as clinically indicated. Monitor AST, ALT and total bilirubin weekly for the month after Itvisma administration and during the corticosteroid taper period. If the patient is clinically stable with unremarkable findings at the end of the corticosteroid taper period, continue to monitor liver function every other week for another month. Tapering of systemic corticosteroids should not be considered until AST/ALT levels are less than 2x ULN (1).

Itvisma has also been associated with thrombocytopenia, peripheral sensory neuropathy, thrombotic microangiopathy (TMA), elevated cardiac troponin I, and risk of tumorigenicity due to AAV vector integration. Monitor platelets before injection and weekly for the first month until platelet counts return to baseline. Consider complete neurologic evaluation and other testing and/or symptom management based on clinical presentation. If clinical signs, symptoms and/or laboratory findings consistent with TMA occur such as hypertension, bruising easily, seizures, or decreased urine output, consult a hematologist and/or nephrologist immediately. Consider cardiac evaluation after Itvisma administration and consult a cardiologist as needed. AAV vector integration carries a theoretical tumor risk, and any tumors should be reported to Novartis Gene Therapies, Inc (1).

The safety and effectiveness of Itvisma in patients less than 2 years of age have not been established (1).

Related policies
Evrysdi, Spinraza, Zolgensma

Policy

This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.

Itvisma may be considered **medically necessary** if the conditions indicated below are met.

Itvisma may be considered **investigational** for all other indications.

Prior-Approval Requirements

Age 2 years of age or older

Diagnosis

Patient must have the following:

1. Spinal Muscular Atrophy (SMA)

AND ALL of the following:

a. Diagnosis confirmed by genetic testing demonstrating bi-allelic mutations in the survival motor neuron 1 (SMN1) gene with **ONE** of the following:
   i. Deletion of both copies of the SMN1 gene **OR**
   ii. Compound heterozygous mutations of the SMN1 gene (defined below):
      a) Pathogenic variant(s) in both copies of the SMN1 gene
      b) Pathogenic variant in 1 copy and deletion of the second copy of the SMN1 gene
b. Baseline anti-adeno-associated virus serotype 9 (AAV9) antibody titers ≤ 1:50
c. Documentation of a genetic test confirming no more than 3 copies of the SMN2 gene
d. Documentation of baseline laboratory assessments for AST, ALT, and total bilirubin
e. Prescribed by a neurologist, neuromuscular specialist, or pediatrician with expertise in treating SMA
f. Patient has not previously received gene therapy for SMA (see Appendix 1)
g. **NO** concurrent use with another Prior Authorization (PA) medication for SMA (see Appendix 2)

Prior – Approval *Renewal* Requirements

None

Policy Guidelines

Pre - PA Allowance

None

Prior - Approval Limits

Quantity 1 injection per lifetime

Duration 1 month

Prior – Approval *Renewal* Limits

None

Rationale

Summary

Itvisma is an AAV vector-based gene therapy indicated for the treatment of spinal muscular atrophy (SMA). Itvisma has a boxed warning regarding serious liver injury. Itvisma has also been associated with thrombocytopenia, peripheral sensory neuropathy, thrombotic microangiopathy (TMA), elevated cardiac troponin I, and risk of tumorigenicity due to AAV vector integration. The safety and effectiveness of Itvisma in pediatric patients less than 2 years of age has not been established (1).

Prior approval is required to ensure the safe, clinically appropriate, and cost-effective use of Itvisma while maintaining optimal therapeutic outcomes.

References

1. Itvisma [package insert]. Bannockburn, IL: Novartis Gene Therapies, Inc.; November 2025.

Policy History

Date	Action
January 2026	Addition to PA
March 2026	Annual review. Per SME, added appendix 2 and specified no concurrent therapy with other PA medications for SMA

Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on March 6, 2026 and is effective on April 1, 2026.

Appendix 1 - List of Gene Therapies for SMA

Generic Name	Brand Name
onasemnogene abeparvovec-brve	Itvisma
onasemnogene abeparvovec-xioi	Zolgensma

Appendix 2 - List of PA medications for SMA

Generic Name	Brand Name
nusinersen	Spinraza
onasemnogene abeparvovec-brve	Itvisma
onasemnogene abeparvovec-xioi	Zolgensma
risdiplam	Evrysdi