FDA approved as of November 24, 2025

itvisma®
(onasemnogene abeparvovec-brve)
suspension for intrathecal injection

Hospital Formulary Review Guide

INDICATION

ITVISMA is indicated for the treatment of spinal muscular atrophy (SMA) in adult and pediatric patients 2 years of age and older with confirmed mutation in *survival motor neuron 1 (SMN1)* gene.

IMPORTANT SAFETY INFORMATION

BOXED WARNING: Serious Liver Injury

Acute serious liver injury can occur with ITVISMA. Hepatotoxicity, with elevated alanine transaminase (ALT) and/or aspartate transaminase (AST) levels, has occurred with ITVISMA. Patients with preexisting hepatic impairment or acute hepatic viral infection may be at higher risk of liver injury. To mitigate potential aminotransferase elevations, administer systemic corticosteroid before and after ITVISMA injection. Prior to ITVISMA injection, assess liver function 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. In case hepatic injury is suspected, further testing is recommended. Promptly consult with a gastroenterologist or hepatologist, as necessary.

FDA, US Food and Drug Administration.

Please see additional Important Safety Information on page 18 and click here for Full Prescribing Information, including Boxed WARNING.

Spinal muscular atrophy overview

SMA is a rare and debilitating neurodegenerative disorder characterized by the irreversible loss of motor neurons[1-3]

SMA is caused by a missing or nonworking *SMN1* gene[3,4]

- *SMN1* is the primary gene responsible for producing the SMN protein, which is critical to the health of motor neurons
- The backup gene, *SMN2*, produces only about 10% to 15% of the SMN protein

It is estimated that there are approximately 9250 people living with SMA in the United States[5]

Treatment goals for children, teens, and adults prioritize incremental gains and maintenance of motor function[6]

Previously, patients aged 2 years and older had access only to treatments which require chronic, repeated administration[7,8]

SMA, spinal muscular atrophy; *SMN2*, *survival motor neuron 2* gene; SMN, survival motor neuron.

Please see Indication and Important Safety Information on page 18 and click here for Full Prescribing Information, including Boxed WARNING.

Product overview

ITVISMA® (onasemnogene abeparvovec-brve) is the only one-time gene therapy for adult and pediatric patients with spinal muscular atrophy aged 2 years and older[9]

ITVISMA was approved by the FDA on November 24, 2025.

Product description

ITVISMA is a suspension of an adeno-associated viral vector-based gene therapy for intrathecal injection. It is a recombinant, self-complementary AAV9 containing a transgene encoding the human SMN protein, under the control of a cytomegalovirus enhancer/chicken β actin hybrid promoter.[9]

ITVISMA has a nominal concentration of 4.0×10^{13} vg/mL. Each vial contains an extractable volume of not less than 3 mL and the excipients 20 mM Tris (pH 8.0), 1 mM $MgCl_2$, 200 mM NaCl, and 0.005% poloxamer 188. ITVISMA is packaged as a sterile suspension and contains no preservative.[9]

Product information

HCPCS code[10]	Miscellaneous J-code J3590 and miscellaneous C-code C9399 until a product-specific code is issued by the Centers for Medicare and Medicaid Services
Package strength[9]	Nominal concentration of 4.0×10^{13} vg/mL. Each vial contains an extractable volume of not less than 3 mL and the excipients 20 mM Tris (pH 8.0), 1 mM $MgCl_2$, 200 mM NaCl, and 0.005% poloxamer 188
Description[10]	**J3590:** Unclassified Biologics **C9399:** Unclassified Drugs or Biologicals
NDC[9]	Single vial: 71894-200-**01** Carton: 71894-200-**02** Before submitting claims, confirm with the patient's health plan which NDC to include

Please contact your RAAD to learn more about site-of-care onboarding

AAV9, adeno-associated virus serotype 9; HCPCS, Healthcare Common Procedure Coding System; $MgCl_2$, magnesium chloride; NaCl, sodium chloride; NDC, National Drug Code; RAAD, Regional Account Associate Director; vg, vector genome.

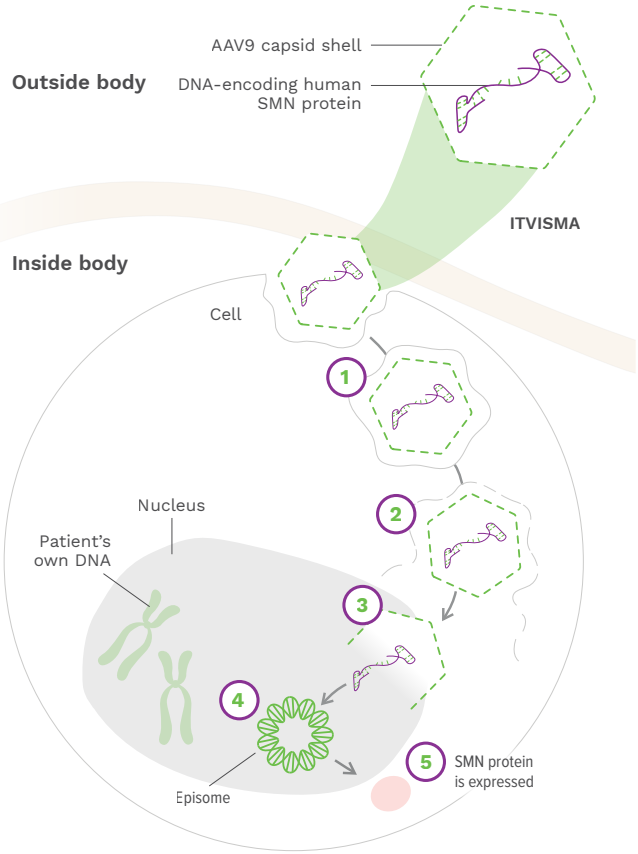
Please see Indication and Important Safety Information on page 18 and click here for Full Prescribing Information, including Boxed WARNING.

Clinical overview

Mechanism of action

ITVISMA® (onasemnogene abeparvovec-brve) is a non-replicating recombinant AAV vector that utilizes AAV9 capsid to deliver a functional copy of the human *SMN1* gene. 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. 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.[9]

1. The AAV9 vector enters motor neurons[9]
2. The AAV9 vector delivers the *SMN* gene to the cell nucleus[9,11]
3. The *SMN* gene is introduced to target cells as recombinant, self-complementary DNA[9]
4. The self-complementary ends form a circular episome that can persist in the nucleus of motor neuron cells. These cells are nondividing[11,12]
5. This results in rapid activation and continuous expression of the *SMN* gene, leading to the production of SMN protein[13]



Click here for more information on ITVISMA

AAV, adeno-associated virus.

FDA approval letter

Our STN: BL 125856/0

BLA APPROVAL
November 24, 2025

Novartis Gene Therapies, Inc.
Attention: Lisa Krueger, PharmD
2275 Half Day Road, Suite 300
Bannockburn, IL 60015

Dear Dr. Krueger:

Please refer to your Biologics License Application (BLA) received March 28, 2025, submitted under section 351(a) of the Public Health Service Act (PHS Act) for onasemnogene abeparvovec-brve.

LICENSING

We have approved your BLA for onasemnogene abeparvovec-brve effective this date. You are hereby authorized to introduce or deliver for introduction into interstate commerce, onasemnogene abeparvovec-brve under your existing Department of Health and Human Services U.S. License No. 2250. Onasemnogene abeparvovec-brve is indicated for the treatment of spinal muscular atrophy (SMA) in adult and pediatric patients 2 years of age and older with confirmed mutation in *survival motor neuron 1 (SMN1)* gene.

The review of this product was associated with the following National Clinical Trial (NCT) numbers: 03381729, 03421977, 04042025, 05089656, 05335876, 05386680.

MANUFACTURING LOCATIONS

Under this license, you are approved to manufacture onasemnogene abeparvovec-brve at your facility located at Novartis Gene Therapies, Inc. in Durham, NC ("GTxNC"). You may label your product with the proprietary name ITVISMA and market it in single-dose vials containing 3 mL product.

ADVISORY COMMITTEE

We did not refer your application to the Cellular, Tissue, and Gene Therapies Advisory Committee because our review of information submitted in your BLA, including the clinical study design and trial results, did not raise concerns or controversial issues that would have benefited from an advisory committee discussion.

U.S. Food & Drug Administration
10903 New Hampshire Avenue
Silver Spring, MD 20993
www.fda.gov

Please see Indication and Important Safety Information on page 18 and click here for Full Prescribing Information, including Boxed WARNING.

FDA approval letter (cont)

DATING PERIOD

The dating period for onasemnogene abeparvovec-brve drug product shall be 24 months from the date of manufacture when stored upright at ≤-60°C. The date of manufacture shall be defined as the date on which the last vial of the drug product is sealed. Following the final sterile filtration, reprocessing by refiltration is allowed, once per lot, under limited conditions (failure of the post-use sterile filter integrity test, loss of integrity of the closed line between the filtered drug product and the filling needle, and setup issues associated with the filling needle assembly not caused by operator error). The dating period for your drug substance shall be 12 months when stored at ≤-60°C.

FDA LOT RELEASE

Please submit protocols showing results of all applicable tests. You may not distribute any lots of products until you receive a notification of release from the Director, Center for Biologics Evaluation and Research (CBER).

BIOLOGICAL PRODUCT DEVIATIONS

You must submit reports of biological product deviations under 21 CFR 600.14. You should identify and investigate all manufacturing deviations promptly, including those associated with processing, testing, packaging, labeling, storage, holding and distribution. If the deviation involves a distributed product, may affect the safety, purity, or potency of the product, and meets the other criteria in the regulation, you must submit a report on Form FDA 3486 to the Director, Office of Compliance and Biologics Quality, electronically through the eBPDR web application or at the address below. Links for the instructions on completing the electronic form (eBPDR) may be found on CBER's web site at https://www.fda.gov/vaccines-blood-biologics/report-problem-center-biologics-evaluation-research/biological-product-deviations :

Food and Drug Administration
Center for Biologics Evaluation and Research
Document Control Center
10903 New Hampshire Ave.
WO71-G112
Silver Spring, MD 20993-0002

MANUFACTURING CHANGES

You must submit information to your BLA for our review and written approval under 21 CFR 601.12 for any changes in, including but not limited to, the manufacturing, testing, packaging or labeling of onasemnogene abeparvovec-brve, or in the manufacturing facilities.

FDA approval letter (cont)

LABELING

We hereby approve the draft content of labeling including the Package Insert submitted under amendment 47, dated November 20, 2025 and the draft package and container labels submitted under amendment 43, dated November 17, 2025.

CONTENT OF LABELING

As soon as possible, but no later than 14 days from the date of this letter, please submit the final content of labeling (21 CFR 601.14) in Structured Product Labeling (SPL) format via the FDA automated drug registration and listing system, (eLIST) as described at http://www.fda.gov/ForIndustry/DataStandards/StructuredProductLabeling/default.htm. Content of labeling must be identical to the Package Insert submitted on November 20, 2025. Information on submitting SPL files using eLIST may be found in the guidance for industry *SPL Standard for Content of Labeling Technical Qs and As* at http://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM072392.pdf.

The SPL will be accessible via publicly available labeling repositories.

PACKAGE AND CONTAINER LABELS

Please electronically submit final printed package and container labels identical to the package and container labels submitted on November 17, 2025, according to the guidance for industry *Providing Regulatory Submissions in Electronic Format — Certain Human Pharmaceutical Product Applications and Related Submissions Using the eCTD Specifications* at https://www.fda.gov/downloads/drugs/guidancecomplianceregulatoryinformation/guidances/ucm333969.pdf.

All final labeling should be submitted as Product Correspondence to this BLA, STN BL 125856/0 at the time of use and include implementation information on Form FDA 356h.

ADVERTISING AND PROMOTIONAL LABELING

You may submit proposed introductory advertising and promotional labeling with Form FDA 2253 to the Advertising and Promotional Labeling Branch at the following address:

Food and Drug Administration
Center for Biologics Evaluation and Research
Document Control Center
10903 New Hampshire Ave.
WO71-G112
Silver Spring, MD 20993-0002

Please see Indication and Important Safety Information on page 18 and click here for Full Prescribing Information, including Boxed WARNING.

FDA approval letter (cont)

You must submit copies of your final advertising and promotional labeling at the time of initial dissemination or publication, accompanied by Form FDA 2253 (21 CFR 601.12(f)(4)).

All promotional claims must be consistent with and not contrary to approved labeling. You should not make a comparative promotional claim or claim of superiority over other products unless you have substantial evidence or substantial clinical experience to support such claims (21 CFR 202.1(e)(6)).

ADVERSE EVENT REPORTING

You must submit adverse experience reports in accordance with the adverse experience reporting requirements for licensed biological products (21 CFR 600.80) and you must submit distribution reports as described in 21 CFR 600.81. In addition to the reporting requirements in 21 CFR 600.80, you must submit adverse experience reports for cases of hepatic failure, thrombotic microangiopathy, and peripheral sensory neuropathy as 15-day expedited reports to the FDA Adverse Event Reporting System (FAERS). Hepatic failure, thrombotic microangiopathy, and peripheral sensory neuropathy reports must be submitted as 15-day expedited reports for three years following the date of product licensure. For information on adverse experience reporting, please refer to the guidance for industry *Providing Submissions in Electronic Format — Postmarketing Safety Reports* at https://www.fda.gov/regulatory-information/search-fda-guidance-documents/providing-submissions-electronic-format-postmarketing-safety-reports and FDA's Adverse Event reporting System website at https://www.fda.gov/drugs/questions-and-answers-fdas-adverse-event-reporting-system-faers/fda-adverse-event-reporting-system-faers-electronic-submissions. For information on distribution reporting, please refer to the guidance for industry *Electronic Submission of Lot Distribution Reports* at https://www.fda.gov/vaccines-blood-biologics/lot-release/lot-distribution-database-ldd.

RARE PEDIATRIC DISEASE PRIORITY REVIEW VOUCHER

Your request for a rare pediatric disease priority review voucher has been denied. You did not qualify for the voucher because your application did not meet the requirements to be a "rare pediatric disease product application" under section 529(a)(4) of the Federal Food, Drug & Cosmetic Act (FD&C Act). FDA has determined that BLA 125856 is not a human drug application for a biological product that contains no active ingredient that has been previously approved in any other application under section 351(a) or 351(k) of the PHS Act. Specifically, BLA 125856 is for a biological product that contains an active ingredient that was previously approved in another application under section 351(a) of the PHS Act. The active ingredient onasemnogene abeparvovec was previously approved on May 24, 2019, in BLA 125694 for ZOLGENSMA.

Please see Indication and Important Safety Information on page 18 and click here for Full Prescribing Information, including Boxed WARNING.

FDA approval letter (cont)

PEDIATRIC REQUIREMENTS

Under the Pediatric Research Equity Act (PREA) (21 U.S.C. 355c), all applications for new active ingredients, new indications, new dosage forms, new dosing regimens, or new routes of administration are required to contain an assessment of the safety and effectiveness of the product for the claimed indication in pediatric patients unless this requirement is waived, deferred, or inapplicable.

Because the biological product for this indication has an orphan drug designation, you are exempt from this requirement.

The biological product was studied in the pediatric patients 2 years of age or older for the intended indication.

POST APPROVAL FEEDBACK MEETING

New biological products qualify for a post approval feedback meeting. Such meetings are used to discuss the quality of the application and to evaluate the communication process during drug development and marketing application review. The purpose is to learn from successful aspects of the review process and to identify areas that could benefit from improvement. If you would like to have such a meeting with us, please contact the Regulatory Project Manager for this application.

Sincerely,

Asha Das -S
Digitally signed by Asha Das -S
Date: 2025.11.24 16:45:39 -05'00'

Asha Das, MD
Acting Director
Office of Clinical Evaluation
Office of Therapeutic Products
Center for Biologics Evaluation and Research

Testing prior to treatment

Prior to treatment with ITVISMA® (onasemnogene abeparvovec-brve), the following tests need to be performed

- Genetic confirmation of SMA diagnosis or historical documentation of prior test[9]
- Baseline testing for the presence of anti-AAV9 antibodies[9]
- Clinical stability in their overall baseline health status (eg, hydration and nutritional status, absence of infection, respiratory status)[9]
- Liver function assessment (clinical examination, AST, ALT, albumin, prothrombin time, PTT, INR, and total bilirubin)[9]
- Creatinine and complete blood count (including hemoglobin and platelet count)[9]
- Verification of the pregnancy status of females of reproductive potential[9]

Prior to dosing

1

Confirm all tests described in the section above have been completed and results indicate that the patient is eligible for treatment with ITVISMA. Assess vaccination status. Vaccination status should be up-to-date prior to ITVISMA administration[9]

2

One day prior to ITVISMA injection, begin administration of systemic corticosteroids equivalent to oral prednisolone at 1 mg per kg of body weight per day (mg/kg/day) for a total of 30 days. Do not stop systemic corticosteroids abruptly. After the 30-day period, taper or continue prednisolone (or equivalent) as needed according to the clinical status and liver function testing[9]

3

Ensure the patient is clinically stable in their overall baseline health status[9]

ALT, alanine aminotransferase; AST, aspartate aminotransferase; INR, international normalized ratio; PTT, partial thromboplastin time.

Please see Indication and Important Safety Information on page 18 and click here for Full Prescribing Information, including Boxed WARNING.

Dosing and administration

ITVISMA® (onasemnogene abeparvovec-brve) is a single-dose intrathecal injection and should only be administered intrathecally using a lumbar puncture by health care professionals (eg, interventional radiologist or neurologist) experienced in performing lumbar punctures.[9]

Patients previously treated with onasemnogene abeparvovec-xioi should not be treated with ITVISMA.[9]

Due to the increased risk of serious systemic immune response, administer ITVISMA to patients who are clinically stable in their overall baseline health status (eg, hydration and nutritional status, absence of infection, respiratory status) prior to administration. Postpone ITVISMA in patients with active or recent infections, until the infection has resolved, and the patient is clinically stable. Clinical signs or symptoms of infection should not be evident at the time of ITVISMA injection.[9]

The recommended dosage of ITVISMA is 1.2×10^{14} vg[9]

Dosing and administration (cont)

Vial preparation

ITVISMA® (onasemnogene abeparvovec-brve) should be prepared aseptically[9]

Ensure you have the required supplies for injection, detailed in the table on page 13[9]

Thaw ITVISMA in the refrigerator for approximately 4 hours, or at room temperature for approximately 1 hour. If thawed in the refrigerator, remove from refrigerator on day of dosing[9]

Do not use ITVISMA unless thawed[9]

Prior to intrathecal injection, ITVISMA should be brought to room temperature[9]

When thawed, ITVISMA is a clear to slightly opaque, colorless to faint white liquid, free of particles. After withdrawal of ITVISMA from the vial, a visual inspection is required. DO NOT use if particulates, cloudiness, or discoloration are visible[9]

DO NOT SHAKE[9]

Immediately prior to dosing, draw the content from the vial into the syringe, remove air from syringe, confirm the dose volume of 3 mL in the syringe, cap syringe and deliver to patient injection location[9]

Once dose is drawn into the syringe, it may be held in the refrigerator at 2-8 °C (36-46 °F) for up to 24 hours, including a 5-hour maximum time out-of-refrigeration allowance within the 24-hour period. Discard the vector-containing syringe if not injected within this time period[9]

DO NOT REFREEZE[9]

Please see Indication and Important Safety Information on page 18 and click here for Full Prescribing Information, including Boxed WARNING.

Dosing and administration (cont)

Component materials compatible with ITVISMA® (onasemnogene abeparvovec-brve)[9]

COMPONENT	MATERIAL OF CONSTRUCTION
18-G to 19-G needle for withdrawal, maximum 1.5" long	Stainless steel
5-mL to 10-mL syringe[a]	Polypropylene
Syringe cap[a]	Polypropylene or polyethylene or methacrylate-acrylonitrile-butadiene-styrene
22-G to 27-G spinal needle, maximum 150 mm long	Stainless steel

[a]Not to be manufactured with PVC, BPA, DEHP, or latex.

Procedural preparation instructions[9]

- Consider sedation if indicated by the patient's clinical status
- Consider imaging techniques to guide intrathecal injection of ITVISMA
- Evaluate patient prior to and after intrathecal injection for conditions that may contraindicate lumbar puncture or increase procedural risk to prevent serious complications

Administration[9]

- Prior to administration, remove 3 mL of CSF using a lumbar puncture needle to create space for injection volume
- Administer ITVISMA as an intrathecal bolus injection over approximately 1 to 2 minutes through the lumbar puncture needle
- Place the patient in the Trendelenburg position (head down at 30 degrees for 15 minutes). Adjust patient positioning and duration based on the patient's clinical status to enhance distribution
- Follow standard post-lumbar puncture care protocols

Monitoring following treatment with ITVISMA[9]

Monitor liver function (AST, ALT, total bilirubin) weekly for the month after ITVISMA injection and during the corticosteroid taper period (28 days or longer if needed).

- If the patient is clinically stable with unremarkable findings (normal clinical exam, total bilirubin, and ALT and AST levels below 2 × ULN) at the end of the corticosteroid taper period, continue to monitor liver function every other week for another month

Monitor platelet counts weekly for the first month and as clinically indicated until platelet counts return to baseline.

Treatment protocols are at the discretion of the clinician and site of care

BPA, bisphenol-A; CSF, cerebrospinal fluid; DEHP, bis(2-ethylhexyl) phthalate; PVC, polyvinylchloride; ULN, upper limit of normal.

Please see Indication and Important Safety Information on page 18 and click here for Full Prescribing Information, including Boxed WARNING.

Distribution and acquisition

Distributed by	Novartis Gene Therapies, Inc. **www.novartis.com** **www.itvisma-hcp.com**
Product name	ITVISMA®
Established name	onasemnogene abeparvovec-brve
NDC information[9]	**Vial NDC: 71894-200-01** Single-dose vial with an extractable volume of not less than 3 mL, containing 1.2×10^{14} vg **Carton NDC: 71894-200-02** The dimensions of the carton are 2.559" x 2.559" x 3.626"
Wholesale acquisition cost	**$2,586,629.70** as of November 24, 2025
Average sales price[14]	The average sales price for ITVISMA is not yet available. The Centers for Medicare and Medicaid Services publishes average sales price-based payment limits on a 2-quarter lag
Product availability	ITVISMA is available through buy-and-bill and specialty pharmacies and is available to ship. The following page provides an overview of the limited distribution network for ITVISMA
Storage and handling[9]	Product is shipped and delivered frozen (≤–60 °C [–76 °F]) in a single-dose clear vial. Upon receipt, immediately place the carton in a refrigerator at 2-8 °C (36-46 °F). ITVISMA is stable for 14 days from receipt when stored at 2-8 °C (36-46 °F) DO NOT REFREEZE. Must use within 14 days of receipt
Product returns	If you have questions about ITVISMA returns, please contact Novartis Pharmaceuticals Corporation by phone at **1-800-526-0175**, or email **tradeoperations.phuseh@novartis.com**. For returns of ITVISMA damaged in shipment, please contact your distributor
Patient support program	Your practice and patients will have access to a Novartis Patient Support™ team committed to providing the support your patients need when they need it, including: • Dedicated assistance with insurance and reimbursement • Personalized support for your patients on therapy • Single point of contact for you and your patients • **Click here** to learn more about Novartis Patient Support
Additional information	Novartis Medical information **medinfo.novartispharmaceuticals.com**

Please see Indication and Important Safety Information on page 18 and click here for Full Prescribing Information, including Boxed WARNING.

Distribution and acquisition (cont)

ITVISMA® (onasemnogene abeparvovec-brve) is available through buy and bill or specialty pharmacy through the following sources

SPECIALTY DISTRIBUTOR	CONTACT INFORMATION	WEBSITE
CuraScript SD®	Phone: **1-866-263-8464** Fax: **1-866-353-5117**	**https://curascriptsd.com**

SPECIALTY PHARMACY	CONTACT INFORMATION	WEBSITE
Accredo® Specialty Pharmacy	Phone: **1-800-803-2523** Fax: **1-877-329-4605**	**https://www.accredo.com**
Orsini Specialty Pharmacy	Phone: **1-800-697-5048** Fax: **1-877-471-5704**	**https://www.orsini.com**
Axium/Farmacia Doral Specialty Pharmacy (Puerto Rico only)	Phone: **1-844-355-4191** Fax: **1-800-546-2163**	**https://www.axiumpr.com**

Prior to ordering product, your site of care needs to be onboarded. To initiate the onboarding process, contact Novartis Patient Support at 1-855-441-4363

Frequently asked questions

1 What are the most common adverse reactions for ITVISMA® (onasemnogene abeparvovec-brve)?

The most common adverse reactions that occurred in ≥2% of patients treated with ITVISMA were upper respiratory tract infection, pyrexia, upper gastrointestinal symptoms, hepatic enzymes increased, headache, dizziness, pain in extremity, thrombocytopenia, and sensory disturbance.[9]

2 Does treatment with ITVISMA impact a patient's vaccine schedule?

Adjust a patient's vaccination schedule to accommodate concomitant corticosteroid administration prior to and following ITVISMA injection. Certain vaccines, such as measles, mumps, and rubella (MMR) and varicella, are contraindicated for patients on a substantially immunosuppressive steroid dose (ie, ≥2 weeks of daily receipt of 20 mg or 2 mg/kg body weight of prednisone or equivalent).[9]

3 What monitoring is required following ITVISMA?

Following treatment with ITVISMA, monitor[9]

- Liver function (AST, ALT, total bilirubin) weekly for the month after ITVISMA injection (or longer based on clinical outcomes) and during the corticosteroid taper period (over the next 28 days or longer if needed). If the patient is clinically stable with unremarkable findings (normal clinical exam, total bilirubin, and ALT and AST levels below 2 × ULN) at the end of the corticosteroid taper period, continue to monitor liver function every other week for another month
- Platelet counts weekly for the first month and as clinically indicated until platelet counts return to baseline
- Please refer to the Full Prescribing Information for additional monitoring details

4 What is the recommended time frame to submit an order?

It is recommended to submit an order as soon as possible. The recommended time frame to submit an order is by 12 PM ET 5 days before the requested delivery date. Before placing your first order, please reach out to your RAAD for your site of care to be properly onboarded.

5 Where can I find more information about coding and billing?

For more information, **click here** to access the ITVISMA HCP Coding and Billing Guide.

HCP, health care professional.

Please see Indication and Important Safety Information on page 18 and click here for Full Prescribing Information, including Boxed WARNING.

Frequently asked questions (cont)

6 What is the cost per patient?

The list price for ITVISMA® (onasemnogene abeparvovec-brve) is $2,586,629.70. For insured patients, out-of-pocket costs vary based on coverage structure defined by the insurance plan.

7 When do you expect a product-specific J-code?

Product-specific J-codes are typically assigned within a year following drug approval.[10] Once ITVISMA receives a product-specific J-code, you can no longer bill to the miscellaneous J-code.

In the meantime, bill using the **miscellaneous J-code J3590** or **miscellaneous C-code C9399** for ITVISMA.[10]

Appropriate codes may vary by setting of care and by payor. Providers are encouraged to reach out to payors to confirm appropriate coding procedures.

For more information on related diagnosis and testing codes, CPT® codes, and NDC codes for ITVISMA, you can **click here** to download the HCP Coding and Billing Guide.

CPT, Current Procedural Terminology.

References: 1. Lin CW, Kalb SJ, Yeh WS. Delay in diagnosis of spinal muscular atrophy: a systematic literature review. *Pediatr Neurol.* 2015;53(4):293-300. **2.** Glascock J, Sampson J, Haidet-Phillips A, et al. Treatment algorithm for infants diagnosed with spinal muscular atrophy through newborn screening. *J Neuromuscul Dis.* 2018;5(2):145-158. **3.** Kolb SJ, Kissel JT. Spinal muscular atrophy. *Neurol Clin.* 2015;33(4):831-846. **4.** MedlinePlus. Spinal muscular atrophy. Updated December 27, 2023. Accessed October 8, 2025. https://medlineplus.gov/spinalmuscularatrophy.html **5.** Cure SMA. State of SMA. Published March 31, 2025. Accessed October 31, 2025. https://www.curesma.org/wp-content/uploads/2025/03/State-of-SMA-Report2024_vWeb.pdf **6.** Cure SMA. Voice of the patient report. Published January 10, 2018. Accessed November 7, 2025. https://www.curesma.org/wp-content/uploads/2018/01/SMA-VoP-for-publication-1-22-2018.pdf **7.** SPINRAZA. Prescribing information. Biogen. **8.** EVRYSDI. Prescribing information. Genentech, Inc. **9.** ITVISMA. Prescribing information. Novartis Gene Therapies, Inc. **10.** Centers for Medicare and Medicaid Services. Billing and coding: hospital outpatient drugs and biologicals under the Outpatient Prospective Payment System (OPPS). Updated December 26, 2024. Accessed October 8, 2025. https://www.cms.gov/medicare-coverage-database/view/article.aspx?articleId=55913 **11.** Colella P, Ronzitti G, Mingozzi F. Emerging issues in AAV-mediated in vivo gene therapy. *Mol Ther Methods Clin Dev.* 2017;8:87-104. **12.** Haggerty DL, Grecco GG, Reeves KC, Atwood B. Adeno-associated viral vectors in neuroscience research. *Mol Ther Methods Clin Dev.* 2019;17:69-82. **13.** Mendell JR, Al-Zaidy S, Shell R, et al. Single-dose gene-replacement therapy for spinal muscular atrophy. *N Engl J Med.* 2017;377(18):1713-1722. **14.** Centers for Medicare and Medicaid Services. Average sales price (ASP) quarterly publication process frequently asked questions. Published January 17, 2025. Accessed November 19, 2025. https://www.cms.gov/files/document/frequently-asked-questions-faqs-asp-data-collection.pdf

Please see Indication and Important Safety Information on page 18 and click here for Full Prescribing Information, including Boxed WARNING.

Indication and Important Safety Information

itvisma®
(onasemnogene abeparvovec-brve)
suspension for intrathecal injection

INDICATION

ITVISMA is indicated for the treatment of spinal muscular atrophy (SMA) in adult and pediatric patients 2 years of age and older with confirmed mutation in *survival motor neuron 1 (SMN1)* gene.

IMPORTANT SAFETY INFORMATION

BOXED WARNING: Serious Liver Injury

Acute serious liver injury can occur with ITVISMA. Hepatotoxicity, with elevated alanine transaminase (ALT) and/or aspartate transaminase (AST) levels, has occurred with ITVISMA. Patients with preexisting hepatic impairment or acute hepatic viral infection may be at higher risk of liver injury. To mitigate potential aminotransferase elevations, administer systemic corticosteroid before and after ITVISMA injection. Prior to ITVISMA injection, assess liver function 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. In case hepatic injury is suspected, further testing is recommended. Promptly consult with a gastroenterologist or hepatologist, as necessary.

WARNINGS AND PRECAUTIONS

Thrombocytopenia
Transient decreases in platelet counts were observed within the first week after ITVISMA administration. Monitor platelet counts before ITVISMA injection and on a regular basis afterwards until platelet counts return to baseline.

Peripheral Sensory Neuropathy
Peripheral sensory neuropathy has occurred with ITVISMA administration with onset seen at approximately 3 weeks post-injection in clinical studies. Consider complete neurologic evaluation and other testing and/or symptom management based on the patient's clinical presentation.

Thrombotic Microangiopathy
Thrombotic microangiopathy (TMA) may occur with ITVISMA administration and can result in life-threatening or fatal outcomes. Monitor platelet counts on a regular basis following ITVISMA injection, as well as signs and symptoms of TMA. Consult a hematologist and/or nephrologist immediately to manage TMA as clinically indicated.

Elevated Cardiac Troponin I

Increases in cardiac troponin I levels have occurred following ITVISMA administration. Consider cardiac evaluation after ITVISMA administration and consult a cardiologist as needed.

AAV Vector Integration and Risk of Tumorigenicity
There is a theoretical risk of tumorigenicity due to integration of AAV vector DNA into the genome. The clinical relevance of individual integration events is unknown, but it is acknowledged that individual integration events could potentially contribute to a risk of tumorigenicity. Report cases of tumor development in patients who received ITVISMA to Novartis Gene Therapies, Inc. at 1-833-828-3947.

ADVERSE EVENTS

The most common adverse reactions that occurred in ≥2% of patients treated with ITVISMA were upper respiratory tract infection, pyrexia, upper gastrointestinal symptoms, hepatic enzymes increased, headache, dizziness, pain in extremity, thrombocytopenia, and sensory disturbance.

Please click here for full Prescribing Information.

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