For US Health Care Professionals

zolgensma® (onasemnogene abeparvovec-xioi) suspension for intravenous infusion

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

Product Ordering Pathways

This guide outlines product support and the ordering process for ZOLGENSMA and ITVISMA through the Novartis Limited Distribution Network. The Regional Account Associate Director (RAAD) is your designated resource for supporting health care professionals (HCPs) throughout the process. Additional assistance is available through Novartis Patient Support™.

Indication and Important Safety Information for ZOLGENSMA

INDICATION

ZOLGENSMA is an adeno-associated virus (AAV) vector-based gene therapy indicated for the treatment of pediatric patients less than 2 years of age with spinal muscular atrophy (SMA) with bi-allelic mutations in the *survival motor neuron 1* (*SMN1*) gene.

Limitations of Use

The safety and effectiveness of repeat administration or the use in patients with advanced SMA (eg, complete paralysis of limbs, permanent ventilator dependence) has not been evaluated with ZOLGENSMA.

IMPORTANT SAFETY INFORMATION

BOXED WARNING: Serious Liver Injury and Acute Liver Failure

Cases of acute liver failure with fatal outcomes have been reported. Acute serious liver injury, acute liver failure, and elevated aminotransferases can also occur with ZOLGENSMA. Patients with preexisting liver impairment may be at higher risk. Prior to infusion, assess liver function of all patients by clinical examination and laboratory testing. Administer systemic corticosteroid to all patients before and after ZOLGENSMA infusion. Continue to monitor liver function for at least 3 months after infusion, and at other times as clinically indicated. If acute serious liver injury or acute liver failure is suspected, promptly consult a pediatric gastroenterologist or hepatologist.

Indication and Important Safety Information for ITVISMA

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.

Please see additional Important Safety Information for ZOLGENSMA on page 7 and click here for full Prescribing Information, including Boxed WARNING.

Please see additional Important Safety Information for ITVISMA on page 8 and click here for full Prescribing Information, including Boxed WARNING.

Novartis Patient Support™

Novartis Patient Support supports you and your patients at every step of the treatment journey, to help ensure your patients receive the one-time gene replacement therapy in a timely manner.

The Novartis Patient Support team includes a Case Coordinator, who can provide insurance support, financial support, and ongoing support for eligible patients prescribed ZOLGENSMA® (onasemnogene abeparvovec-xioi) or ITVISMA® (onasemnogene abeparvovec-brve), including

- Answering questions related to SMA and treatment
- Explaining the steps before and after treatment
- Helping navigate the insurance and reimbursement process, including benefits verification and prior authorization and appeals support
- Identifying financial support options and eligibility
- Tracking the medication from prescription to delivery to the site of administration

Enrollment

Step 1

- Download the Novartis Patient Support Start Form from either **zolgensma-hcp.com/resources** or **itvisma-hcp.com/support/novartis-patient-support**

Step 2

- Submit the completed Start Form to Novartis Patient Support by

Fax to **1-855-951-4363**

OR

Online at **zolgensma-enrollment.com** or **itvisma-enrollment.com**

If the Start Form is missing a caregiver's or patient's signature, the signed Patient Authorization and Additional Consents Form can be submitted with the Start Form

The Patient Authorization and Additional Consents Form can be downloaded from either **zolgensma-hcp.com/resources** or **itvisma-hcp.com/support/novartis-patient-support**

Please contact Novartis Patient Support for more information at 1-855-441-4363

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Product Ordering Pathways

ZOLGENSMA® (onasemnogene abeparvovec-xioi) and ITVISMA® (onasemnogene abeparvovec-brve) are available to purchase through 2 ordering pathways, CuraScript SD® for buy and bill or through a limited network of specialty pharmacies

Specialty Distributor (Buy and Bill)

- CuraScript SD® Specialty Distribution
  Phone: **1-866-263-8464**

Limited Network Specialty Pharmacies

- Accredo® Specialty Pharmacy
  Phone: **1-800-803-2523**
- Orsini Specialty Pharmacy
  Phone: **1-800-697-5048**
- Axium/Farmacia Doral Specialty Pharmacy (Puerto Rico only)
  Phone: **1-844-355-4191**

RAADs provide dedicated support to HCPs and sites of care, including assistance in onboarding new sites of care. For more information, contact

Name:

Email:

Phone:

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Buy-and-Bill Pathway

ZOLGENSMA® (onasemnogene abeparvovec-xioi) or ITVISMA® (onasemnogene abeparvovec-brve) can be ordered directly from CuraScript SD® Specialty Distribution, the authorized specialty distributor, through the buy-and-bill process. In this process, your organization will purchase and bill for the product, its administration, and related professional services. HCPs may enroll patients in Novartis Patient Support, which can help you navigate the access and reimbursement process.

1 Product Ordering and Delivery

- Your institution will order the product directly from CuraScript SD® Specialty Distribution
- The site of care will submit a product-specific order form to CuraScript SD® Specialty Distribution at least 48 hours prior to the desired delivery date for ZOLGENSMA and 5 business days prior to the desired delivery date for ITVISMA
  - Reach out to your RAAD to obtain the order form
  - For ZOLGENSMA, the patient's weight must be confirmed with the institution in a timely manner to ensure effective treatment[1]
- Your RAAD can assist the institution with confirming the target treatment date and HCP
- CuraScript SD® Specialty Distribution will then submit the order to Novartis
- Product will be delivered to the site of care in a GPS-enabled, temperature-monitoring, dry ice, smart shipping container using white-glove delivery to the specific individual provided by the site of care to sign for the shipment
  - ZOLGENSMA is shipped and delivered frozen (≤–60 °C [–76 °F]) in clear vials to the site of care[1]
  - ITVISMA is shipped and delivered frozen (≤–60 °C [–76 °F]) in a single-dose, clear vial to the site of care[2]
- Novartis and the logistics service provider will manage and monitor the shipment and confirm receipt with the site of care

2 Receipt, Handling, Storage, and Administration

- Product should be stored in a refrigerator at 2-8 °C (36-46 °F)[1,2]
- Product must be used within 14 days of receipt[1,2]
- **Click here** for more information on the preparation and administration of ZOLGENSMA
- **Click here** for more information on the preparation and administration of ITVISMA

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Buy-and-Bill Pathway (cont)

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Billing and Reimbursement

Submit a claim to the insurance provider using

ZOLGENSMA® (onasemnogene abeparvovec-xioi)

Product-specific J-code: J3399[3]

- **Click here** to access the **ZOLGENSMA Coding and Billing Guide** for more information

ITVISMA® (onasemnogene abeparvovec-brve)

Product-specific J-code: J3405[4]

- **Click here** to access the **ITVISMA Coding and Billing Guide** for more information

Please contact your RAAD to assist with billing and reimbursement support

Specialty Pharmacy Pathway

Specialty pharmacies authorized by Novartis will work with your organization, Novartis Patient Support, and the patient or caregiver to obtain the appropriate weight-based dose of ZOLGENSMA® (onasemnogene abeparvovec-xioi) in a timely manner. ITVISMA® (onasemnogene abeparvovec-brve) is not a weight-based medication.

The specialty pharmacy will manage the reimbursement for the product, and the HCP/site of care will bill for the administration and professional services.

1 Specialty Pharmacy Coordination and Ordering

- If the patient is enrolled, Novartis Patient Support will partner with the specialty pharmacy to confirm patient eligibility, prescription information, and the target infusion or injection date
- If the patient is not enrolled, the HCP can send all information needed directly to the specialty pharmacy
- The HCP needs to provide the specialty pharmacy with a prescription
- The specialty pharmacy will contact the patient or caregiver and the HCP to confirm the target infusion or injection date, site of care details, and obtain agreement to ship the product
- For ZOLGENSMA, the patient's weight must be confirmed with the institution in a timely manner to ensure effective treatment[1]
- The specialty pharmacy will work directly with CuraScript SD® Specialty Distribution to order the product

2 Product Delivery

- Product will be delivered to the site of care in a GPS-enabled, temperature-monitoring, dry ice, smart shipping container using white-glove delivery to the specific individual provided by the site of care to sign for the shipment
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Indication and Important Safety Information for ZOLGENSMA

zolgensma® (onasemnogene abeparvovec-xioi) suspension for intravenous infusion

INDICATION

ZOLGENSMA is an adeno-associated virus (AAV) vector-based gene therapy indicated for the treatment of pediatric patients less than 2 years of age with spinal muscular atrophy (SMA) with bi-allelic mutations in the *survival motor neuron 1* (*SMN1*) gene.

Limitations of Use

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IMPORTANT SAFETY INFORMATION

BOXED WARNING: Serious Liver Injury and Acute Liver Failure

Cases of acute liver failure with fatal outcomes have been reported. Acute serious liver injury, acute liver failure, and elevated aminotransferases can also occur with ZOLGENSMA. Patients with preexisting liver impairment may be at higher risk. Prior to infusion, assess liver function of all patients by clinical examination and laboratory testing. Administer systemic corticosteroid to all patients before and after ZOLGENSMA infusion. Continue to monitor liver function for at least 3 months after infusion, and at other times as clinically indicated. If acute serious liver injury or acute liver failure is suspected, promptly consult a pediatric gastroenterologist or hepatologist.

WARNINGS AND PRECAUTIONS

Systemic Immune Response

Patients with underlying active infection, either acute or chronic uncontrolled, could be at an increased risk of serious systemic immune response. Administer ZOLGENSMA to patients who are clinically stable in their overall health status (eg, hydration and nutritional status, absence of infection). Postpone ZOLGENSMA in patients with infections until the infection has resolved and the patient is clinically stable.

Thrombocytopenia

Transient decreases in platelet counts, some of which met the criteria for thrombocytopenia, were typically observed within the first 2 weeks after ZOLGENSMA infusion. Monitor platelet counts before ZOLGENSMA infusion and on a regular basis for at least 3 months afterwards.

Thrombotic Microangiopathy

Cases of thrombotic microangiopathy (TMA) were reported to occur generally within the first 2 weeks after ZOLGENSMA infusion. TMA can result in life-threatening or fatal outcomes. Obtain baseline creatinine and complete blood count before ZOLGENSMA infusion. Following infusion, monitor platelet counts closely as well as other signs and symptoms of TMA. Consult a pediatric hematologist and/or pediatric nephrologist immediately to manage as clinically indicated.

Elevated Troponin I

Increases in cardiac troponin I levels have occurred following ZOLGENSMA infusion. Consider cardiac evaluation after ZOLGENSMA infusion 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. Cases of tumor have been reported in patients who received ZOLGENSMA post-approval; a causal relationship has not been established based on tumor analysis. In some cases, limited information was available. Report cases of tumor development in patients who received ZOLGENSMA to Novartis Gene Therapies, Inc. at 1-833-828-3947.

Infusion-Related Reactions

Infusion-related reactions, including hypersensitivity reactions and anaphylaxis, have occurred with ZOLGENSMA infusion. Signs and symptoms may include rash, urticaria, vomiting, dyspnea, respiratory symptoms, and/or alterations in heart rate and blood pressure. Monitor patients during and after treatment with ZOLGENSMA. If an infusion-related reaction occurs, interrupt ZOLGENSMA infusion and administer supportive treatment to manage the infusion-related reaction as appropriate. Infusion of ZOLGENSMA may be resumed based on clinical assessment.

ADVERSE REACTIONS

The most commonly observed adverse reactions (incidence ≥5%) in clinical studies were elevated aminotransferases and vomiting.

Please click here for full Prescribing Information.

Indication and Important Safety Information for ITVISMA

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.

References: 1. ZOLGENSMA. Prescribing information. Novartis Gene Therapies, Inc. **2.** ITVISMA. Prescribing information. Novartis Gene Therapies, Inc. **3.** Centers for Medicare and Medicaid Services. HCPCS quarterly update. 2026. Accessed May 18, 2026. https://www.cms.gov/medicare/coding-billing/healthcare-common-procedure-system/quarterly-update **4.** Centers for Medicare and Medicaid Services. Healthcare Common Procedure Coding System (HCPCS) application summaries and coding determinations. Accessed May 18, 2026. https://www.cms.gov/files/document/2026-hcpcs-application-summary-quarter-1-2026-drugs-biologicals.pdf

NOVARTIS

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