NOVARTIS

November 24, 2025

Dear SMA Community:

We are proud to share this news with the SMA community—on November 24, 2025 the US Food and Drug Administration (FDA) approved ITVISMA® (onasemnogene abeparvovec-brve) suspension, for intrathecal injection, for adults and children 2 years of age and older with spinal muscular atrophy (SMA).

This approval marks a significant step forward, expanding the reach of gene replacement therapy to patients. It is the result of years of determination by patients and families, community participation in clinical trials, unwavering efforts from advocacy groups, rigorous research by scientists and clinicians, and collaboration with regulators and Novartis teams around the world.

We are working diligently to provide you with additional information about ITVISMA, including on access to treatment and available support. In the meantime, please see below for frequently asked questions, and check www.itvisma.com for the latest updates and information in the coming weeks.

The most common side effects reported in clinical trials with ITVISMA were infections of the nose, throat, and sinuses, fever, upper stomach or digestive symptoms, increased liver enzymes, headache, dizziness, pain in extremity, low platelet counts, and sensory disturbance. Please continue reading for important safety information, and please see the accompanying full Prescribing Information.

The approval of ITVISMA represents a new chapter, expanding treatment options. We remain committed to standing alongside you and supporting families at every stage of the SMA journey.

Sincerely,
The Novartis Team

Indication and Important Safety Information for ITVISMA® (onasemnogene abeparvovec-brve) suspension for intrathecal injection

INDICATION

What is ITVISMA?

ITVISMA is a prescription gene therapy used to treat adults and children 2 years of age and older with spinal muscular atrophy (SMA). ITVISMA is given as a one-time intrathecal injection.

IMPORTANT SAFETY INFORMATION

What is the most important safety information I should know about ITVISMA?

- ITVISMA can increase liver enzyme levels and cause hepatotoxicity.
- Patients will receive an oral corticosteroid medication before and after ITVISMA injection and will undergo regular blood tests to monitor liver function.

- Patients and caregivers should contact the patient's doctor immediately if the patient's skin and/or whites of the eyes become yellowish, if the patient misses a dose of corticosteroid or vomits it up, or if the patient experiences a decrease in alertness.

What should I watch for before and after injection with ITVISMA?

- Infections before or after ITVISMA injection can lead to more serious complications. Patients, caregivers, and close contacts of the patient should follow infection prevention procedures. Patients and caregivers should contact the patient's doctor immediately if the patient experiences any signs of a possible infection such as coughing, wheezing, sneezing, runny nose, sore throat, or fever.
- Decreased platelet counts could occur following injection with ITVISMA. Seek immediate medical attention if the patient experiences unexpected bleeding or bruising.
- Peripheral sensory neuropathy has occurred with ITVISMA administration. Patients and caregivers should contact the patient's doctor right away if the patient experiences numbness, tingling, prickling, or pain in the arms, hands, legs, and/or feet.
- Decreased blood platelet and red blood cell counts, sudden kidney change, and increased bruising or bleeding, which could be signs of thrombotic microangiopathy (TMA), can occur. Patients and caregivers should seek immediate medical attention if the patient experiences unexpected bruising or bleeding, seizures, or decreased urine output.
- There is a theoretical risk of tumor development with gene therapies such as ITVISMA. Patients and caregivers should contact the patient's doctor and Novartis Gene Therapies, Inc. at 1-833-828-3947 if a tumor develops.

What do I need to know about vaccinations and ITVISMA?

- Patients and caregivers should consult the patient's doctor about vaccinations and ITVISMA.
- Patients and caregivers should talk with the patient's doctor to determine if adjustments to the patient's vaccination schedule are necessary during corticosteroid use.
- Protection against influenza and respiratory syncytial virus (RSV) is recommended, and vaccination status should be up-to-date prior to ITVISMA administration.

What do I need to know about contraception and egg or sperm donation and ITVISMA?

- Women of childbearing potential should use an effective method of contraception and refrain from egg donation for 6 months following ITVISMA injection.
- Men capable of fathering a child should use a barrier method of contraception and refrain from sperm donation for 3 months following ITVISMA injection.

What are the possible or likely side effects of ITVISMA?

- The most common adverse reactions that occurred in patients treated with ITVISMA were upper respiratory tract infection, fever, upper gastrointestinal symptoms, increased liver enzymes, headache, dizziness, pain in extremity, low platelet counts, and sensory disturbance.

The safety information provided here is not comprehensive. Talk to the patient's doctor about any side effects that bother the patient or that don't go away.

You are encouraged to report suspected side effects by contacting the FDA at 1-800-FDA-1088 or www.fda.gov/medwatch, or Novartis Gene Therapies, Inc. at 1-833-828-3947.

Please see the Full Prescribing Information.

Frequently Asked Questions About ITVISMA

How is ITVISMA administered?
ITVISMA (onasemnogene abeparvovec-brve) is an adeno-associated virus (AAV) vector-based gene therapy used to treat patients with SMA patients 2 years of age and older. It is given as a one-time treatment administered by, or under the direction of, health care professionals experienced in giving intrathecal injections via an intrathecal injection, an injection that is given directly into the spinal canal. It is given as a single, fixed dose, irrespective of the patient's weight.

Who is eligible to receive treatment with ITVISMA?
ITVISMA is for the treatment of children 2 years of age and older, teens, and adults living with spinal muscular atrophy (SMA). Patients should discuss specific eligibility criteria with their health care provider.

What data for ITVISMA supported the approval?
The approval of ITVISMA is based on data from the registrational Phase lll STEER study (NCT05089656) and supported by the open label Phase lllb STRENGTH study (NCT05386680). The STEER study evaluated patients who had not received previous treatment for SMA, and the STRENGTH study evaluated patients who had discontinued another treatment prior to enrolling in the study.

What support is available to patients who have been prescribed ITVISMA?
As part of our commitment to developing innovative medicines for people with rare diseases, Novartis offers resources to help eligible patients access their treatment.

Novartis Patient Support™ (NPS) is a comprehensive program that provides personalized support to assist patients in navigating their health insurance coverage and identifying financial assistance options, while also offering educational resources to help them get started on treatment and guide them along the way.

Resources include:

- Dedicated one-on-one Case Coordinator phone support to help patients and their caregivers navigate the process before and after treatment
- Access and reimbursement support, including calling the health plan on the patient's behalf to check insurance coverage for therapy, including non-emergency travel support, prior authorization support, and appeals support

- Information on financial support options, including the ITVISMA CopayAssist™ Program for eligible patients
- Resources about living with SMA, taking ITVISMA, and finding supportive communities

Where can I get answers to my questions on ITVISMA?
For more information about ITVISMA, visit www.itvisma.com. Patients and caregivers should contact their health care provider to answer any questions about whether ITVISMA might be appropriate for them.

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