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

PICTURE YOUR ONE DOSE LIFE

The only one-time gene replacement therapy for kids 2 years of age and older, teens, and adults with SMA

Lab tests and administration of oral corticosteroids are required before and after injection.

SMA, spinal muscular atrophy.

Andrew C., 22 years old
Sports journalist and student
Living with SMA

Andrew hasn't received ITVISMA. Individuals shown were compensated for their time.

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.

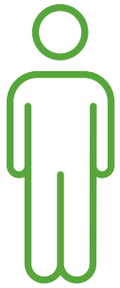
What is the most important safety information I should know about ITVISMA?
- ITVISMA can increase liver enzyme levels and cause hepatotoxicity.
- Patients will receive an oral corticosteroid medication before and after ITVISMA injection and will undergo regular blood tests to monitor liver function.
- 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.

Please see additional Important Safety Information on page 11 and Full Prescribing Information.

PICTURE

WHAT ONE DOSE CAN DO

ITVISMA is an SMA treatment with*:



- ✗ **No refills**
- ✗ **No recurring injections**
- ✗ **No ongoing reauthorizations**
- ✓ **One dose designed to work continuously**

What could a one-dose treatment mean to you?

SMA, spinal muscular atrophy.
*Corticosteroids are required before and after treatment and are administered orally (which may require refills and reauthorization) or via injection if oral corticosteroids are not tolerated or effective. Talk to your doctor.

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.

Please see the Indication and additional Important Safety Information on page 11 and Full Prescribing Information.

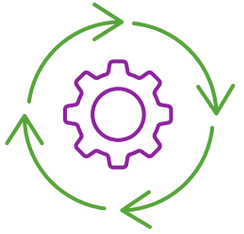
HOW

ITVISMA WORKS

One-time ITVISMA delivers a new, working *SMN1* gene

ITVISMA® (onasemnogene abeparvovec-brve) is a **gene replacement therapy** that delivers a new, working *survival motor neuron 1* (*SMN1*) gene to replace the function of the missing or nonworking *SMN1* gene in the body with only one dose. **Here's how:**

The **new, working *SMN1* gene** is placed inside a delivery vehicle called a vector.



This vector delivers the new, working gene to motor neuron cells, enabling **continuous production** of survival motor neuron (SMN) protein.

By delivering this new, working gene, ITVISMA can **stop the progressive loss of motor neurons**, which are essential to muscle function.

ITVISMA is designed to work continuously to increase the amount of SMN protein.

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

- 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.

Please see the Indication and additional Important Safety Information on page 11 and Full Prescribing Information.

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

EVERY POINT COUNTS

A single dose was shown to stop SMA progression and improve motor function in people with no prior treatment

Efficacy was based on the change in baseline Hammersmith Functional Motor Scale – Expanded (HFMSE) and Revised Upper Limb Module (RULM) scores at ~1 year.

The main study that evaluated ITVISMA was called the STEER study. The purpose of this study was to establish the efficacy and safety of ITVISMA in people with SMA, aged 2 to <18 years old, who had never been on SMA treatment.

Who participated in the study?

126 people participated in the study.

75 of these participants were given ITVISMA, and 51 were part of a sham-control group (which means no drug was injected).

All 126 participants were able to sit but never able to walk independently.

Andrew lives with SMA and hasn't received ITVISMA. He was compensated for his time.
SMA, spinal muscular atrophy.

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

- 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.

Please see the Indication and additional Important Safety Information on page 11 and Full Prescribing Information.

Scarlette lives with SMA and hasn't received ITVISMA.
She was compensated for her time.

The main study: STEER

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

A single dose was shown to stop SMA progression and improve motor function

Results in people with no prior treatment:

2.39
point improvement in overall motor function

People treated with ITVISMA saw a **2.39-point improvement in their motor function** score, as measured by HFMSE (vs a 0.51-point improvement in the sham-control group), which **may be a clinically significant increase at ~1 year**.

Secondary result from the STEER study:

We cannot make conclusions from the following data. They are presented for observational purposes. These endpoints did not meet statistical significance as outlined in the study.

- **People also saw a 2.44-point increase in their RULM score**, a scale used to measure the strength and function of their hands and arms (vs a 0.92-point change in the sham-control group) at ~1 year

Find out more about the HFMSE and RULM rating scales and results with ITVISMA.

HFMSE, Hammersmith Functional Motor Scale – Expanded; RULM, Revised Upper Limb Module; SMA, spinal muscular atrophy.

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.

Please see the Indication and additional Important Safety Information on page 11 and Full Prescribing Information.

The supporting study: STRENGTH

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

Results in people who changed treatment

The supporting study, known as STRENGTH, was an open-label study with no comparator group. This means that in this study all participants received ITVISMA, and participants and doctors both knew what treatment was being given. The results of this type of study could be influenced by the expectations of the patients and doctors. We cannot make conclusions from the following data.

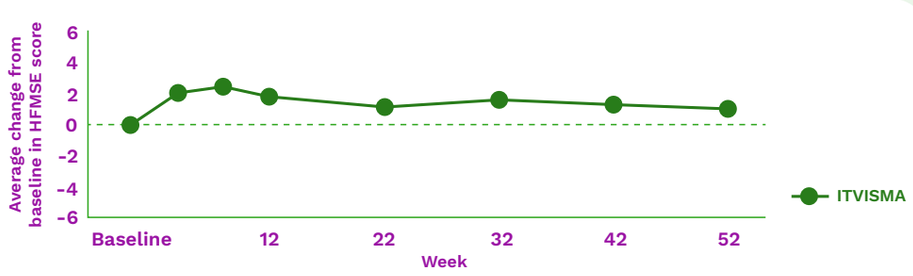
The purpose of this study was to establish the safety and efficacy of ITVISMA in people with SMA aged 2 to <18 years old who had previously been treated with other SMA therapies. Efficacy was a secondary endpoint in the study.

Who participated in the study?

All 27 participants were able to sit but not able to walk independently. Participants had previously taken SPINRAZA® (nusinersen) or EVRYSDI® (risdiplam) and stopped those treatments.

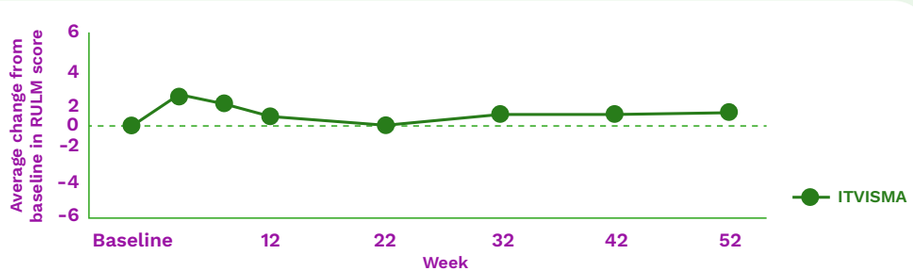
Change in HFMSE and RULM scores

Change in motor function
from the beginning to the end of the study (1 year)*



Change in motor function was measured by HFMSE scores, which assessed people's ability to perform activities such as sitting, rolling, and crawling.

Change in hand and arm function
from the beginning to the end of the study (1 year)*



Change in hand and arm function was measured by RULM scores, which assessed people's ability to perform activities such as picking up and placing items and pressing buttons.

HFMSE, Hammersmith Functional Motor Scale – Expanded; RULM, Revised Upper Limb Module; SMA, spinal muscular atrophy.
*The STRENGTH study looked at the change in HFMSE and RULM scores at 1 year.
EVRYSDI® is a registered trademark of Genentech USA, Inc.
SPINRAZA® is a registered trademark of Biogen.

What do I need to know about vaccinations and ITVISMA? (cont)

- Protection against influenza and respiratory syncytial virus (RSV) is recommended, and vaccination status should be up-to-date prior to ITVISMA administration.

Please see the Indication and additional Important Safety Information on page 11 and Full Prescribing Information.

VIEW THE

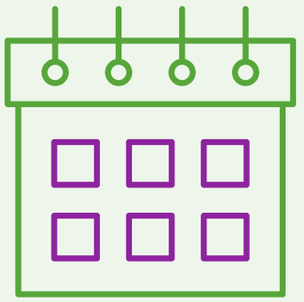
SAFETY PROFILE

The safety profile of ITVISMA was studied in people new to treatment and in people who stopped a previous treatment*

Across 2 clinical studies, ITVISMA has been studied in 153 people living with spinal muscular atrophy (SMA). At the time of administration, ages ranged from 2 to <18 years old.

- The most frequently experienced adverse reactions (adverse reactions seen in ≥2% of people, or were more common with ITVISMA compared to the sham-control group) in the main study were upper respiratory tract infection, fever, stomach-related symptoms, elevated liver enzymes, headache, dizziness, limb pain, low blood platelet count, and sensory disturbance
- When safety was evaluated in people who changed to ITVISMA from either SPINRAZA® (nusinersen) or EVRYSDI® (risdiplam), no additional safety events were found compared to the main study. Other side effects may be possible

People can experience side effects with ITVISMA. Start a conversation with your health care team for more information on the safety profile of ITVISMA and if it may be right for you.



After you receive your dose, your doctor will monitor you closely.†

*The safety data described in this section includes ITVISMA in 2 clinical studies: STEER, a randomized, sham-controlled study, which evaluated the safety of ITVISMA in 126 patients with SMA, and STRENGTH, an open-label, single-arm study, which evaluated the safety of ITVISMA in 27 patients with SMA who were previously treated with nusinersen (at least 4 months washout) or risdiplam (at least 15 days washout).
†People with SMA should continue to see their neurologist and other specialists as needed.
EVRYSDI® is a registered trademark of Genentech USA, Inc.
SPINRAZA® is a registered trademark of Biogen.

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.

Please see the Indication and additional Important Safety Information on page 11 and Full Prescribing Information.

YOUR ONE-DOSE

TREATMENT STARTS HERE

These are the next steps to take to help with a smooth treatment journey

Important lab tests

A doctor will schedule lab tests to determine if you or your loved one are eligible for ITVISMA. Ask the doctor about getting these tests. Before starting treatment, ensure that you know your vaccination status and that it is up-to-date.

Doctor submits the Novartis Patient Support™ Start Form

Ask the doctor to submit a Novartis Patient Support Start Form so you can access additional support before, during, and after treatment.

Working with your insurance

Connect with the Novartis Patient Support team to learn how they can help.

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.

Please see the Indication and additional Important Safety Information on page 11 and Full Prescribing Information.

Andrew lives with SMA and hasn't received ITVISMA.
Individuals shown were compensated for their time.

Scarlette lives with SMA and hasn't received ITVISMA.
Individuals shown were compensated for their time.

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

CONTINUING DOWN THE

PATH TO TREATMENT

Preparing for treatment day

Download the Step-by-Step Guide to keep important information in one easy-to-find place.

Treatment day

Here are some important things to remember:

- While the injection may only take 1 to 2 minutes, treatment will take longer and sedation or imaging may be needed. Be sure to talk to your doctor about planning for injection day
- The corticosteroid dose should be taken following the injection as directed by your doctor. Be sure to confirm future corticosteroid doses that you or your loved one will need to take, and schedule follow-up appointments and lab work with the doctor

Moving forward after treatment

You and your doctor will schedule follow-up appointments over the next few weeks for follow-up lab work. Monitoring is needed for at least 3 months.

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.

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.

Please see additional Important Safety Information on page 11 and Full Prescribing Information.

YOU'VE GOT

SUPPORT THROUGHOUT YOUR JOURNEY

Whether it's a question about the insurance process, how to start on treatment, or ways you can get more involved in the spinal muscular atrophy (SMA) community, there's a resource available to help you find the answer.

Enroll in Novartis Patient Support™

Our dedicated Case Coordinators offer one-on-one guidance on financial support, the insurance process, and more. For information, visit our support page or call 1-855-441-4363 Monday through Friday (8:00 AM to 8:00 PM ET).

Connect with the broader SMA community

There's always room for more when it comes to support. Learn more about SMA support groups, organizations, and upcoming events that can provide additional information and a broader SMA community.

"It's important for your doctor to know your needs and your wants, because life with SMA is unwavering."
– Andrew C., living with SMA

Andrew was compensated for his time.

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

- 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.

Please see the Indication and additional Important Safety Information on page 11 and Full Prescribing Information.

INDICATION AND IMPORTANT SAFETY INFORMATION

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

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.

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.

PICTURE YOUR

ONE DOSE LIFE

Lab tests and administration of oral corticosteroids are required before and after injection.

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

Scarlette lives with SMA and hasn't received ITVISMA.
Individuals shown were compensated for their time.

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.

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.

Please see additional Important Safety Information on page 11 and the Full Prescribing Information.

NOVARTIS

Novartis Pharmaceuticals Corporation
East Hanover, New Jersey 07936-1080

12/25

FA-11530434