

WINTER 2026

SMA Community Update from Novartis

Dear SMA Community:

We hope you are enjoying the winter months and looking forward to the warmer days of spring!

In November 2025, Novartis announced the US Food and Drug Administration (FDA) approved ITVISMA[®] (onasemnogene abeparvovec-brve) suspension, for intrathecal injection (an injection into the spine), for children 2 years and older, teens, and adults with spinal muscular atrophy (SMA), representing a one-time gene replacement therapy option. Novartis is grateful to the SMA community and its partners for their ongoing engagement, insights, and participation in clinical trials. For the latest updates and more information, including the [ITVISMA Doctor Discussion Guide](#), please visit www.ITVISMA.com. Please continue reading for Indication and Important Safety Information, and please see the accompanying full [Prescribing Information](#) including Boxed WARNING.

In this Community Update, read on to learn more about:

- [An on-demand ITVISMA webinar](#)
- [Engagement with the SMA community](#)
- [Cure SMA's Adaptive Sports and Recreation webinars](#)
- [The latest from SMAsing My Limits](#)
- [How to share your SMA experience with Novartis](#)

We look forward to continuing to provide timely updates as they become available and seeing you in person at the Annual SMA Conference this summer.

With gratitude,
Your Novartis Team



Earlier this year, the first US ITVISMA[®] delivery took place. We're honored to share a moment from that day.

ITVISMA Webinar Held in December and Available on Demand



Please note: this webinar is intended for US audiences only. This webinar was recorded by Cure SMA and is available on Cure SMA's YouTube channel.

On December 11th, 2025, Cure SMA and Novartis hosted a community webinar on the FDA approval of ITVISMA[®] (onasemnogene abeparvovec-brve). We were glad to welcome over 400 attendees from the SMA community to learn more about this treatment option. Thank you to Cure SMA for inviting Novartis to bring this information to the community.

If you were unable to attend or are interested in learning more, please click [here](#) to view a recording of the live session.

Please continue reading for Indication and Important Safety Information, and please see the accompanying full [Prescribing Information](#) including Boxed WARNING.

Engaging With the SMA Community

Rooted in connection and shared learning, Cure SMA's Summit of Strength events bring the SMA community together for a day focused on education, support, and engagement. Novartis proudly participated and sponsored Summit of Strength events across the country in 2025, supporting Cure SMA's mission to strengthen and connect the SMA community.

Introducing Our Newest Patient and Community Liaison (PCL)!

We're excited to welcome our newest PCL, Brett, who brings 15+ years of rare disease advocacy experience, including nearly a decade in biopharma focused on neuromuscular conditions.

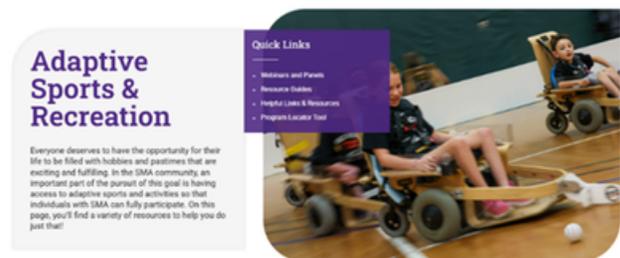
Brett and the other PCLs look forward to meeting you at upcoming Summit of Strength and Walk-n-Roll events!



Learn More About Cure SMA's Adaptive Sports and Recreation Program

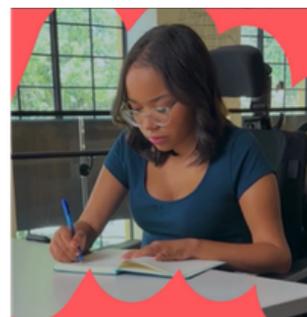
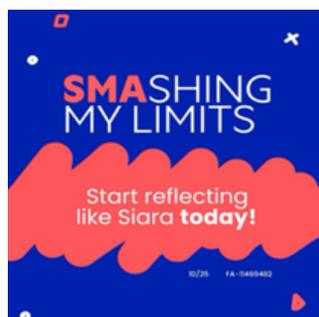
Let's get moving—together! Novartis is proud to be the presenting sponsor of **Cure SMA's Adaptive Sports & Recreation Program**, which highlights opportunities for children, teens, and adults with SMA to explore adaptive sports and recreation. Everyone deserves the opportunity for their life to be filled with hobbies and pastimes that are exciting and fulfilling—and within the SMA community, that means having access to adaptive sports and activities that enable full participation.

The program launched with 2 community webinars held in December and January, with an additional session coming up on February 26th. A new [Adaptive Sports & Recreation webpage](#) is also now live on the Cure SMA website, bringing together webinar recordings, details on upcoming sessions, and curated resources designed to support exploration, participation, and connection across the SMA community.



Reflecting on the Latest From SMASHing My Limits

You may have seen [Siara in our recent Instagram clip](#)—SMASHing limits and reflecting on her SMA journey. Now it's your turn. ❤️ Explore this [reflection resource](#) with prompts to help you recognize your own accomplishments.



Share Your SMA Story With Us!



We know that each SMA journey is unique and would like the opportunity to learn from you. If you would like to share your SMA experience with Novartis, please email us at SMApartners@yuzuzest.com.

If you are under 18 and would like to participate, your parent or guardian will need to provide consent for you. Novartis will never share your information or ask you to participate in activities without your consent.

Indication and Important Safety Information for ITVISMA®

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.

Please keep reading for additional Important Safety Information on the next page.

Important Safety Information for ITVISMA[®] (cont.)

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](#).

