SMA Community Update from Novartis Gene Therapies:
May 2023

Dear SMA Community:

We have enjoyed seeing many of you at Summits of Strength and the Walk-n-Rolls, and look forward to being with you at the next Cure SMA Annual Conference in June. We will gather and work together toward the common goal of creating a positive impact for individuals with SMA and their families.

In this community update we are pleased to share Plain Language Summaries for recently presented data and clinical updates.

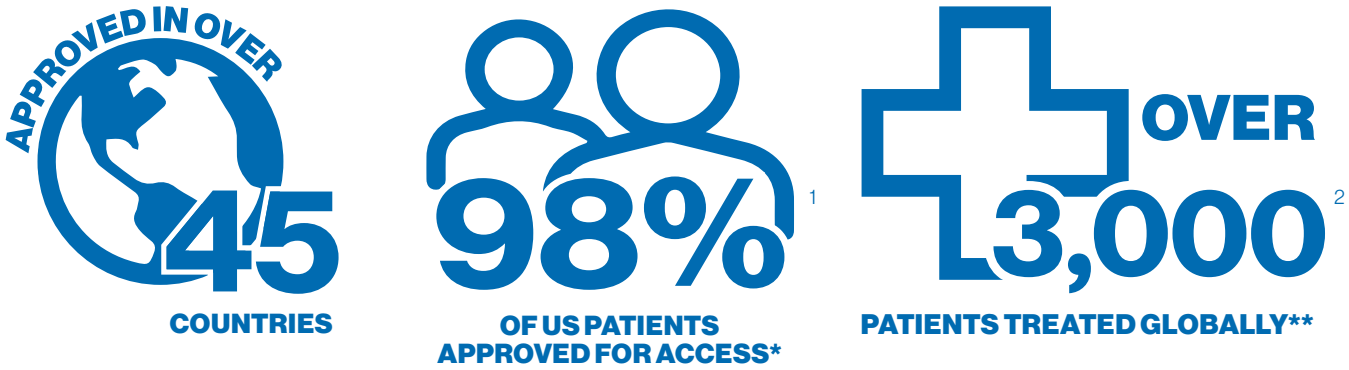
Thank you for your continued partnership, your trust, and your support.

With gratitude,
YOUR NOVARTIS GENE THERAPIES TEAM

Connecting with the Community

We are proud to be a sponsor of the Cure SMA 2023 Annual SMA Conference in June and are excited to be gathering again in person.
We invite you to stop by the Novartis activity room for your Mickey ears and have your questions answered at our booth!

Access Snapshot for ZOLGENSMA® (onasemnogene abeparvovec-xioi) suspension, for intravenous infusion



*Insurance approval rate based on data from 24 May 2019-30 Sep 2021, all patients <2 years of age for whom a payer decision was known
**As of January 2023, including clinical trials, commercially, and through the managed access programs

Plain Language Summaries

Click on the links below to access Plain Language Summaries of our latest research in non-technical language that is intended for non-clinician audiences.

1. Long-Term Follow-Up of Onasemnogene Abeparvovec Gene Therapy in Symptomatic Patients with Spinal Muscular Atrophy Type 1[3]
2. Seroprevalence and Half-life of Pre-existing Anti–adeno-associated Virus Serotype 9 Antibodies in Neonates[4]

You can also read the latest **Novartis News** on Zolgensma long-term data demonstrating sustained durability up to 7.5 years post-dosing.

SMA Clinical Trial Program

Completed Clinical Studies

START

Phase 1 study that evaluated safety and efficacy of ZOLGENSMA® in symptomatic patients

STR1VE

Phase 3 study that evaluated safety and efficacy of ZOLGENSMA® in symptomatic patients

SPR1NT

Phase 3 study that evaluated safety and efficacy of ZOLGENSMA® in presymptomatic patients

STRONG

Phase 1 study that evaluated safety and tolerability of investigational intrathecal gene therapy (OAV101)

Investigational Studies

SMART

Phase 3b study evaluating safety, tolerability, and efficacy of ZOLGENSMA® in patients with SMA weighing ≥8.5 kg and ≤21 kg. For the latest information, please visit clinicaltrials.gov

STRENGTH

Phase 3b, open-label study evaluating safety and tolerability of investigational intrathecal OAV101 in patients aged 2-12 years with SMA after discontinuing treatment with nusinersen or risdiplam. Recruitment has begun. For the latest information, please visit clinicaltrials.gov

STEER

Phase 3 study evaluating efficacy, safety, and tolerability of investigational intrathecal OAV101 in patients with type 2 SMA. STEER includes treatment naive patients aged ≥2 to <18 years, able to sit, but who have never walked. Recruitment has begun. For the latest information, please visit clinicaltrials.gov or the STEER Clinical Trial Webpage on Novartis.com

Long-Term Follow-Up Studies

LT-001

A long-term follow-up study of patients from the START clinical trial for continuous safety monitoring for up to 15 years

LT-002

A long-term follow-up study of patients from the STR1VE, SPR1NT and STRONG clinical trials for continuous monitoring of safety as well as monitoring of continued efficacy and durability of response

SPECTRUM

A long-term follow-up study of patients from OAV101 IT and IV clinical trials for assessment of safety and efficacy, for 15 years after OAV101 administration. For the latest information, please visit clinicaltrials.gov

Please keep reading for Indication and Important Safety Information, and please see accompanying **Full Prescribing Information**, including Boxed Warning

Indication and Important Safety Information for ZOLGENSMA® (onasemnogene abeparvovec-xioi)

What is ZOLGENSMA?

ZOLGENSMA is a prescription gene therapy used to treat children less than 2 years old with spinal muscular atrophy (SMA). ZOLGENSMA is given as a one-time infusion into a vein. ZOLGENSMA was not evaluated in patients with advanced SMA.

What is the most important information I should know about ZOLGENSMA?

- ZOLGENSMA can increase liver enzyme levels and cause acute serious liver injury or acute liver failure which could result in death.
- Patients will receive an oral corticosteroid before and after infusion with ZOLGENSMA and will undergo regular blood tests to monitor liver function.
- Contact the patient's doctor immediately if the patient's skin and/or whites of the eyes appear 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 infusion with ZOLGENSMA?

- Infections before or after ZOLGENSMA infusion can lead to more serious complications. Caregivers and close contacts with the patient should follow infection prevention procedures. 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 infusion with ZOLGENSMA. Seek immediate medical attention if the patient experiences unexpected bleeding or bruising.
- Thrombotic microangiopathy (TMA) has been reported to generally occur within the first two weeks after ZOLGENSMA infusion. Seek immediate medical attention if the patient experiences any signs or symptoms of TMA, such as unexpected bruising or bleeding, seizures, or decreased urine output.

What do I need to know about vaccinations and ZOLGENSMA?

- Talk with the patient's doctor to decide if adjustments to the vaccination schedule are needed to accommodate treatment with a corticosteroid.
- Protection against influenza and respiratory syncytial virus (RSV) is recommended and vaccination status should be up-to-date prior to ZOLGENSMA administration. Please consult the patient's doctor.

Do I need to take precautions with the patient's bodily waste?

- Temporarily, small amounts of ZOLGENSMA may be found in the patient's stool. Use good hand hygiene when coming into direct contact with patient body waste for one month after infusion with ZOLGENSMA. Disposable diapers should be sealed in disposable trash bags and thrown out with regular trash.

What are the possible or likely side effects of ZOLGENSMA?

The most common side effects that occurred in patients treated with ZOLGENSMA were elevated liver enzymes and vomiting.

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 833-828-3947. Please see the Full Prescribing Information.

References

1. Data on file. Novartis Gene Therapies, Inc. 2021. **2.** Data on file. Novartis Gene Therapies, Inc. 2023. **3.** Mendell JR, Wigderson M, et al., Long-Term Follow-Up of Onasemnogene Abeparvovec Gene Therapy in Symptomatic Patients with Spinal Muscular Atrophy Type 1. Poster presented at: Muscular Dystrophy Association Clinical & Scientific Conference; March 19–22, 2023; Dallas, TX. **4.** Van Olden RW, Lo Bianco C, et al., Seroprevalence and Half-life of Pre-existing Anti–adeno-associated Virus Serotype 9 Antibodies in Neonates. Poster presented at: Muscular Dystrophy Association Clinical & Scientific Conference; March 19–22, 2023; Dallas, TX.

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