SMA Community Update from Novartis Gene Therapies:
September 2024

Dear SMA Community:

It was wonderful to have been able to join so many of you at the Cure SMA Annual Conference and SMA Research & Clinical Care Meeting in Austin this year, and we look forward to seeing some of you again at the Cure SMA Chapter and Leadership Meeting. We greatly appreciate your insights and partnership!

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

With gratitude,
YOUR NOVARTIS GENE THERAPIES TEAM

Connecting with the Community

This year we have been proud to sponsor both the Cure SMA Summits of Strength and Walk-n-Rolls. It has been a pleasure to meet so many of you at these events and we look forward to seeing more of you at the remaining events this year.

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

APPROVED IN OVER **54** **COUNTRIES***

98%[1] **OF US PATIENTS APPROVED FOR ACCESS****

OVER 3,500[2] **PATIENTS TREATED GLOBALLY*****

* As of July 2024
**Data derived from the OneGene Program, a patient support service offered by Novartis Gene Therapies (May 2019-Sep 2021); all patients <2 years of age for whom a payer decision was known
***As of August 2023 including clinical trials, commercially, and through the managed access programs

Thank you to the community of ZOLGENSMA families who have been part of our clinical trials and long-term follow-up studies. Your continued participation is greatly appreciated.

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

SMA Clinical Trial Program

Completed Clinical Studies

SMART
Phase 3b study that evaluated safety, tolerability, and efficacy of ZOLGENSMA® in patients with SMA weighing ≥8.5 kg and ≤21 kg

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

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

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

Ongoing Studies

STRENGTH *†
Phase 3b, open-label study evaluating safety and tolerability of investigational intrathecal OAV101 in patients aged 2-<18 years with SMA after discontinuing treatment with nusinersen or risdiplam. 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. 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 ZOLGENSMA® and investigational intrathecal OAV101 clinical trials for assessment of safety and efficacy, for 15 years after administration. For the latest information, please visit clinicaltrials.gov

*OAV101 intrathecal (IT) formulation is not approved for use, and its efficacy and safety have not been established
† Intrathecal (IT) administration is performed via an injection into the spinal canal

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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 t he patient experiences any signs or symptoms of TMA, such as unexpected bruising or bleeding, seizures, or decreased urine output.
- There is a theoretical risk of tumor development with gene therapies such as ZOLGENSMA. Contact the patient's doctor and Novartis Gene Therapies, Inc. (1-833-828-3947) if a tumor develops.

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 1-833-828-3947. 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.

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