



Dear SMA Community,

We have exciting news to share with you—a major milestone has been reached in the fight against spinal muscular atrophy (SMA)! The US Food and Drug Administration (FDA) has approved ZOLGENSMA® (onasemnogene abeparvovec-xioi) for the treatment of children less than 2 years old with SMA.

This monumental day represents years of collaboration and the perseverance of caregivers and patients, patient advocacy groups, researchers, healthcare professionals, treatment teams, AveXis employees, and the FDA. Because of this collaboration, families now have a new treatment option that targets the genetic root cause of SMA with a one-time-only gene therapy.

“It has been a privilege to work together with the SMA community on this journey to bring ZOLGENSMA to children with SMA. Your partnership has been invaluable at every step,” said Dave Lennon, president of AveXis. “I would like to extend a special thank you to all of the families who participated in the clinical studies—and to those who continue to be a part of our ongoing studies—as we make every effort to assist more people living with SMA. Without you, we would not be here.”

We are working diligently to provide you with additional information about ZOLGENSMA, including how to start treatment and the dedicated support available before, during, and after treatment. Please check [ZOLGENSMA.com](https://www.zolgensma.com) for the latest updates and information.

It is important to understand that ZOLGENSMA can cause acute serious liver injury. In the clinical studies of ZOLGENSMA, the most common side effects were elevated liver enzymes and vomiting.

Everyone in the SMA community should be proud of their contributions. The approval of ZOLGENSMA is just the beginning, and we look forward to continuing our partnership and supporting you at every step of the SMA journey.

Sincerely,
The AveXis Team

[Click here](#) for Frequently Asked Questions.

Please see the Indication and Important Safety Information, including Boxed Warning, on page 2 and the accompanying [Full Prescribing Information](#).

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 the vein. ZOLGENSMA was not evaluated in patients with advanced SMA.

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

- Liver enzymes could become elevated and cause acute serious liver injury in children who receive ZOLGENSMA.
- 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, or if the patient misses a dose of the corticosteroid or vomits it up.

What should I watch for before and after infusion with ZOLGENSMA?

- Viral respiratory infections before or after ZOLGENSMA infusion can lead to more serious complications. Contact the patient's doctor immediately if you see signs of a possible viral respiratory 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 a patient experiences unexpected bleeding or bruising.

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 respiratory syncytial virus (RSV) is recommended.

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 bodily waste for up to 1 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 AveXis at 833-828-3947.

Please see the [Full Prescribing Information](#).

Frequently Asked Questions About ZOLGENSMA

How is ZOLGENSMA administered?

ZOLGENSMA® (onasemnogene abeparvovec-xioi) is a prescription gene therapy used to treat children less than 2 years old with SMA. It is given intravenously (IV) over the course of 60 minutes at treatment centers that specialize in administering ZOLGENSMA. Children should not be redosed with ZOLGENSMA. Children infused with ZOLGENSMA should receive an oral corticosteroid 24 hours before the ZOLGENSMA infusion and every day after for at least 30 days to help manage elevated liver enzyme levels. After 30 days, based on a patient's clinical exam, the doctor may gradually lower the dose and eventually stop the steroid. The child's liver enzyme levels will need to be monitored by blood tests for at least 3 months.

What does this mean for older children and adults with SMA?

Our goal is to make ZOLGENSMA available to all SMA patients through a rigorous research and development program. We recognize the needs of the entire SMA population, and we are exploring the necessary steps to study older patients. Clinical study protocols are being developed to study ZOLGENSMA in a broader range of people with SMA. Data from planned and ongoing studies (including STRONG) will help us understand how to best design future studies. Please visit [ClinicalTrials.gov](https://clinicaltrials.gov) for additional information.

What clinical studies for ZOLGENSMA are currently ongoing or planned?

AveXis currently has 5 ongoing studies of SMA and 2 more planned for upcoming initiation. Please visit [ClinicalTrials.gov](https://clinicaltrials.gov) for additional information, including the inclusion and exclusion criteria.

How many people have received ZOLGENSMA?

As of May 2019, more than 130 children have been dosed with ZOLGENSMA.

Will ZOLGENSMA be covered by insurance?

Health insurance coverage for ZOLGENSMA is determined by each individual insurance provider. When a doctor sends in a prescription for ZOLGENSMA with your consent, highly trained people from the OneGene Program, a dedicated resource from AveXis, can work closely with the patient's insurance company to help obtain coverage.

What sites in the United States are infusing ZOLGENSMA?

AveXis is working with pediatric neuromuscular specialists across the country to ensure they know how to administer ZOLGENSMA. You can email treatments@curesma.org to find the nearest treatment center.

When will ZOLGENSMA be approved in other countries?

AveXis is working with other country regulators to ensure they have access to ZOLGENSMA. At this time, we do not have an update on when it will become available, but you can reach out to medinfo@avexis.com if you are in the United States or medinfo.emea@avexis.com if you are in Europe, the Middle East, or Africa to request more information.

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Where can I get answers to my questions on ZOLGENSMA?

Talk to your doctor about ZOLGENSMA® (onasemnogene abeparvovec-xioi) to learn if it is a treatment option for your child. If you have general, nonmedical questions about ZOLGENSMA, the OneGene Program is available to help. This program is a dedicated resource from AveXis that consists of a team of highly trained people who provide support and guidance for families during treatment and beyond. Call 855-441-GENE (4363), Monday-Friday (8 AM-8 PM ET), or visit [ZOLGENSMA.com](https://www.zolgensma.com) for more information.

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