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(onasemnogene abeparvovec-xioi)
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Take the next step for your child

Once you and your child's doctor have discussed the risks and benefits of ZOLGENSMA® (onasemnogene abeparvovec-xioi) and have chosen it as your child's treatment option, the steps on the following pages will help your family understand what to expect next.

Work together with your doctor to find a treatment center by emailing **treatments@curesma.org**.

Starting ZOLGENSMA

Find out if your child is eligible for treatment with ZOLGENSMA › **Connect with the OneGene Program™** › **Prepare for treatment day** › **Treatment day** › **After treatment with ZOLGENSMA**

Support from the OneGene Program

Knowing what to expect and what you can do now will help you and your child prepare for treatment with ZOLGENSMA. Providing support along the way is the OneGene Program. This program is a dedicated resource brought to you by AveXis and is specifically designed to help you and your family. Contact the OneGene Program at 855-441-GENE (4363), Monday-Friday (8 AM-8 PM ET), to learn more.

Please see the Indication and Important Safety Information on page 7 and the accompanying Full Prescribing Information.

1 Find out if your child is eligible for treatment with ZOLGENSMA

ZOLGENSMA® (onasemnogene abeparvovec-xioi) is a prescription gene therapy used to treat children less than 2 years old with spinal muscular atrophy (SMA). It is given as a one-time infusion into the vein. ZOLGENSMA was not evaluated in patients with advanced SMA and is not recommended in premature babies before they reach full-term age. Once you and your child's doctor have chosen treatment with ZOLGENSMA, there are a few steps that must be taken to determine eligibility.

Complete necessary lab tests

- **Confirm a diagnosis of SMA through a genetic test**
  Before ZOLGENSMA can be given, a diagnosis of SMA has to be confirmed. If needed, your doctor will order a genetic test to confirm the survival motor neuron 1 *(SMN1)* gene is missing or nonworking.
- **Your doctor should perform an AAV9 antibody test**
  This test may be required by some insurance providers. An adeno-associated virus 9, or AAV9, antibody test measures the amount of anti-AAV9 antibodies in your child's blood. If your child's immune system has built up a certain level of anti-AAV9 antibodies, he or she may not qualify for ZOLGENSMA right away. If your child's anti-AAV9 antibodies are too high, your doctor has the option to retest. Speak with your doctor about the AAV9 antibody test.
- **Your doctor should perform baseline liver enzyme, platelet, and troponin-I tests**
  Your doctor should perform blood tests to check your child's liver function and to establish baseline levels for other markers in the blood. These measurements before dosing will help your doctor and care team monitor your child's levels after dosing. Your doctor will give you a prescription for an oral corticosteroid to start the day before your child's infusion.

Have your doctor submit a ZOLGENSMA Prescription Form and a Patient Consent Form

While waiting for test results, your doctor may submit a ZOLGENSMA Prescription Form and a Patient Consent Form to the OneGene Program™. This starts the process to assess insurance coverage and authorization requirements and helps you learn about the patient support the OneGene Program offers. You will need to sign the Patient Consent Form to access all of the support provided by the OneGene Program.

Once the Patient Consent Form is completed, a representative from the OneGene Program will call you to discuss the patient support available to you.

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2 Connect with the OneGene Program

The OneGene Program™ is a dedicated resource brought to you by AveXis. It is designed to support and guide you and your family at every step during treatment. After the ZOLGENSMA® (onasemnogene abeparvovec-xioi) Prescription Form and Patient Consent Form are received, a team of highly trained and dedicated people will call you to discuss the variety of patient resources that are available to support your family. You may contact the OneGene Program at 855-441-GENE (4363), Monday-Friday (8 AM-8 PM ET), to learn more.

Some of the resources the OneGene Program provides include:

A **Patient Resource Manager**, who is your main point of contact on behalf of AveXis. This person provides expert guidance and emotional support at every step of your treatment journey, and answers questions about ZOLGENSMA.

A **Case Coordinator**, who acts as a go-between to connect you to resources you may need and assists doctors with insurance processes. Other support a Case Coordinator may provide includes verification of insurance benefits and coordination of financial assistance programs for eligible patients.

A **Field Reimbursement Manager**, who helps your child's doctor navigate insurance requirements and authorizations. Other support includes following your child's prescription through insurance approvals, the pharmacy process, and shipment journey to ensure it is delivered to the right place at the right time.

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Your doctor's office will follow up with you about the status of your insurance coverage approval for ZOLGENSMA.

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3 Prepare for treatment day

If your child is approved for treatment with ZOLGENSMA® (onasemnogene abeparvovec-xioi), your child's doctor and care team will help ensure you know exactly what to expect on the day of treatment and how to prepare. Additionally, your Patient Resource Manager will work with you to understand how he or she can best support your family on treatment day.

If not completed already, your doctor should perform blood tests to check your child's liver function and to establish baseline levels for other markers in the blood. These measurements before dosing will help your doctor and care team monitor your child's levels after dosing. Your doctor will give you a prescription for an oral corticosteroid to start the day before your child's infusion.

Viral respiratory infections before or after ZOLGENSMA infusion can lead to more serious complications. Contact your doctor immediately if you see signs of a possible viral respiratory infection such as coughing, wheezing, sneezing, runny nose, sore throat, or fever.

A course of an oral corticosteroid* should be started the day before infusion with ZOLGENSMA. This helps manage elevated liver enzyme reactions to ZOLGENSMA by the body's immune system.

Talk to your doctor and care team about any family members you would like to have with you on treatment day. You have the option of having your Patient Resource Manager present on the day of treatment to support your family. Ask your Patient Resource Manager to speak with the doctor and care team beforehand so any necessary actions can be addressed before treatment day.

*The specific treatment course for each patient will be determined by the treating doctor. The treatment course is based on several clinical factors and the judgment of the doctor. Caregivers should discuss specific treatment recommendations with their treating doctor.

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If you have any additional questions prior to treatment day, you can reach out to your doctor and care team, Patient Resource Manager, or the support team at the OneGene Program™.

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4 Treatment day

On the day of treatment, your child will be infused with ZOLGENSMA

You should give your child the second dose of the oral corticosteroid on the day of infusion as prescribed to help manage reactions to ZOLGENSMA® (onasemnogene abeparvovec-xioi) by the body's immune system. The actual infusion will take 60 minutes. However, ask your child's doctor or care team for additional details on the schedule for the day. Remember to talk to your doctor and care team about any family members you would like to have with you on treatment day. You may have the option of having your Patient Resource Manager present on the day of treatment to support your family.

Post-treatment plan

Before you leave the hospital, talk with your doctor about post-treatment follow-up and additional monitoring. You will continue to give your child the corticosteroid as prescribed by your doctor. Your doctor will monitor your child's liver function for at least 3 months after infusion through weekly clinical exams and blood tests for the first month and every other week for the second and third months. Your doctor will determine when to gradually reduce the dose of the corticosteroid and when to stop it.

- You should contact your doctor immediately if a dose of the corticosteroid is missed or vomited up
- Talk to your doctor about any side effects

In addition to liver function, your doctor will monitor other markers through blood tests, which may require weekly appointments:

- Weekly platelet counts for the first month and then every other week for the second and third months until platelet counts return to baseline
- Weekly troponin-I for the first month and then monthly for the second and third months until troponin-I level returns to baseline

Now you're ready for the next step, learning about SMA after ZOLGENSMA, and what to do next.

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5 After treatment with ZOLGENSMA

Support that goes further

Your support continues after treatment with ZOLGENSMA® (onasemnogene abeparvovec-xioi). Next steps will include:

- Following up with your child's doctor to evaluate his or her progress
- Speaking with your neuromuscular specialist to review services (like physical and occupational therapy, working with a nutritionist, and meeting with a pulmonologist) to determine what kind of supportive care may be best for your child following treatment
- Sharing information about SMA and ZOLGENSMA with your pediatrician

Continuing your child's SMA care

While ZOLGENSMA replaces the function of your child's missing or nonworking *SMN1* gene, your child still has SMA. That's why it is important to work with your doctor and care team and review supportive care needs. Your child may continue to show signs and symptoms of SMA now or in the future. These may include difficulty swallowing or breathing or muscle weakness. **Call your doctor if you see these, or any other, signs or symptoms.** Additional therapies, accommodations, and support may be needed to help manage your child's SMA and guide his or her ongoing development.

The OneGene Program™ will also reach out periodically after treatment to offer support and answer questions.

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Indication and Important Safety Information

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 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.

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