

CHOOSING TREATMENT FOR YOUR CHILD

After you receive a spinal muscular atrophy (SMA) diagnosis, you may have questions about treatment options for your child. SMA is a progressive disease and in the most severe types, it's critical to get treated quickly.

This guide is designed to help inform and empower you to make the right treatment decision for your child and your family. You can use the statements below to better understand what topics you'd like to discuss with your child's doctor that are related to ZOLGENSMA.



Understanding our treatment plan

I feel confident that we have a treatment plan to treat my child's SMA as quickly as possible:

☐

Agree

☐

Disagree

Here are our family's next steps: _____

Understanding the cause of SMA

I'd rate my knowledge about the *survival motor neuron (SMN) 1* gene and the *SMN2* gene as:

☐

Very Unclear

☐

Somewhat Clear

☐

Crystal Clear

I want to better understand: _____

Understanding gene therapy for SMA

I'd rate my knowledge about how gene therapy works in the treatment of SMA as:

☐

Very Unclear

☐

Somewhat Clear

☐

Crystal Clear

I want to ask my doctor: _____

Understanding ZOLGENSMA

I understand that ZOLGENSMA is a gene therapy designed to deliver SMN protein to my child with only one dose.

Patients need to receive an oral corticosteroid before and after treatment with ZOLGENSMA and undergo blood tests before and after infusion. ZOLGENSMA is not a cure and ongoing SMA care will be required after treatment:

☐

Very Unclear

☐

Somewhat Clear

☐

Crystal Clear

When it comes to making a treatment choice for my child, I understand the time commitment and expectations for administering our chosen therapy:

☐

Yes

☐

No

☐

I could use a little more guidance

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.

Please see additional Important Safety Information on [page 3](#) and the accompanying [Full Prescribing Information](#).



Be Prepared for Your Doctor Visit

Having questions prepared for your next doctor's visit can help ensure you get all the information you need. Below are some questions to consider for your next visit:

- What are your top priorities when deciding on a treatment?
- What concerns do you have regarding your treatment decision for your child?
- What challenges worry you when thinking about managing medication for your child?

Goal setting can be a useful way to determine what treatment option is best suited for your child. When I think about my child's future, my hopes are that...

Ask your doctor about gene therapy with ZOLGENSMA to determine if this option is right for your child.



Pictured left to right: Matthew, Ellie, Montgomery, and Emily. Ellie, diagnosed with SMA before birth, was treated with ZOLGENSMA at 15 days old and is shown here at 9 months of age.

One day can change your child's life

ZOLGENSMA stops the progression of spinal muscular atrophy (SMA) by helping children meet milestones, like sitting, standing, walking on their own, and more.*

[See Results of Clinical Studies](#)

*Results and outcomes vary among children based on several factors, including how far their SMA symptoms progressed prior to receiving treatment.



Talk to your doctor about treating SMA with gene therapy and learn more about ZOLGENSMA at www.ZOLGENSMA.com

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.

Please see the Indication and Important Safety Information on [page 3](#) and the accompanying [Full Prescribing Information](#).

 **zolgensma®**
(onasemnogene
abeparvovec-xioi)
suspension for intravenous infusion

Indication and Important Safety Information

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- 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, during, 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.
- There is a theoretical risk of tumor development with gene therapies such as ZOLGENSMA. Contact the patient's doctor and Novartis Gene Therapies, Inc. at 1-833-828-3947 if a tumor develops.
- Infusion-related reactions may occur during and after ZOLGENSMA infusion. Seek immediate medical evaluation if signs and symptoms of infusion-related reaction occur which may include rash, hives, vomiting, shortness of breath, respiratory symptoms, and/or changes in heart rate and blood pressure.

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.

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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](#).



Learn more about treating SMA with ZOLGENSMA
by visiting www.ZOLGENSMA.com

