

## There is a special child in my life

who has a progressive, rare genetic disease called spinal muscular atrophy (SMA) and was treated with a medication called ZOLGENSMA. The information below and on the following pages will help you learn more about the disease and the treatment my child received.

### Facts about SMA



SMA is a progressive, rare genetic disease, yet it is the **number one genetic cause of infant death**.



people in the United States (or 6 million\* Americans) is a genetic carrier of SMA, and most don't know it.

\*Calculations are based on an estimated US population of 300 million.



SMA affects about **1 in every 10,000 babies** born each year.

#### The cause of SMA

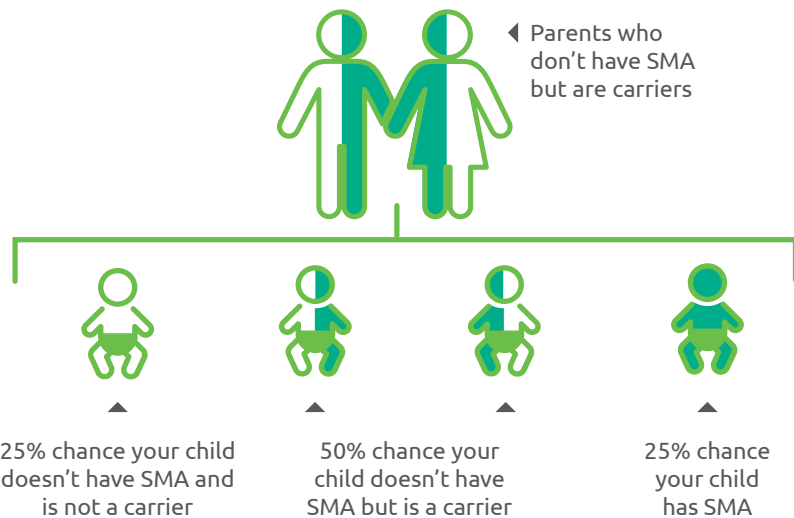
SMA is caused by a missing or nonworking survival motor neuron 1 (*SMN1*) gene. When this gene is missing or not working, motor neuron cells start to die and the cells can't communicate with the muscles and tell them to work properly. Over time, muscles become so weak that things like walking, eating, swallowing, and breathing become harder and harder to do. If left untreated, SMA Type 1 is usually fatal for children by 24 months of age.

#### Most common type of SMA

There are 4 main types of SMA that range in severity, and SMA Type 1, the most common, is very severe.

#### How SMA is inherited

SMA is an autosomal recessive disorder. This means a person must inherit one copy of a nonworking or missing gene from each parent to have the disease.



Please see the Indication and Important Safety Information on page 4 and the accompanying [Full Prescribing Information](#).

## ZOLGENSMA is changing SMA treatment

ZOLGENSMA brings gene therapy to the forefront of SMA treatment. It is a prescription gene therapy used to treat children less than 2 years old with SMA. ZOLGENSMA:



- Is designed to target the genetic root cause of SMA by replacing the function of the missing or nonworking gene with a new, working copy that makes survival motor neuron (SMN) protein. SMN protein keeps motor neuron cells working to preserve strength in the muscles



- Does not change or become a part of the child's DNA



- Is given as a one-time infusion into the vein over 60 minutes

The risks and benefits of ZOLGENSMA in children with advanced SMA (eg, no longer able to move their arms and legs, on permanent breathing support) have not been studied.

### Indication

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.

### Important Safety Information

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.



Please see additional Important Safety Information on page 4 and the accompanying [Full Prescribing Information](#).

## SMA after treatment with ZOLGENSMA

While ZOLGENSMA has replaced the child's missing or nonworking gene with a new, working copy, the child still has SMA. This is because ZOLGENSMA cannot reverse damage already caused by SMA before treatment. Let's learn about the effects SMA can still have on families after treatment with ZOLGENSMA, things you should know, and ways you can help.

- Children treated with ZOLGENSMA may need additional therapies, accommodations, and support to manage their SMA, such as physical therapy, nutritional support, and equipment to help with breathing
- Because SMA affects the muscles used for breathing, it can be difficult to overcome illnesses—even the common cold. As a result, children with SMA should not be around others who are sick. If you or someone you know is ill, notify the child's caregiver right away. In addition, practicing good hygiene around children with SMA is an important part of preventing the spread of germs
- The demands of being a caregiver can feel overwhelming at times, and lending a helping hand can make all the difference. This can include making a meal for the family, offering a sympathetic ear, or babysitting other family members while the child goes to doctor appointments

## How to get involved

Now that you know more about SMA and ZOLGENSMA, it's your turn to share this information with others. You can also learn more about advocacy groups and how you can support the SMA community at [ZOLGENSMA.com](https://www.zolgensma.com). In addition, you can search online for SMA communities that are dedicated to helping families and offering a space to share experiences.

Visit [ZOLGENSMA.com](https://www.zolgensma.com) to learn more about this SMA treatment or to share this information with others.

### Important Safety Information

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.



Please see the Indication and additional Important Safety Information on page 4 and the accompanying [Full Prescribing Information](#).

# 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](http://www.fda.gov/medwatch), or AveXis at 833-828-3947.**

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



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