

# There is a special child in my life

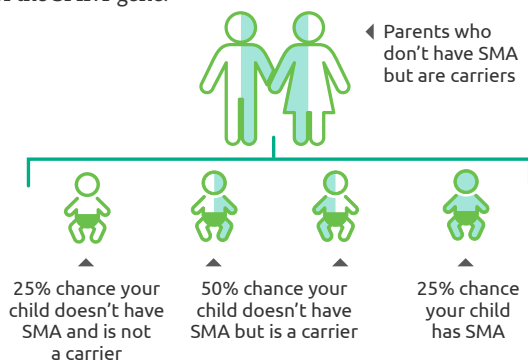
who has a progressive, rare genetic disease called spinal muscular atrophy (SMA) and was treated with a medication called ZOLGENSMA<sup>®</sup> (onasemnogene abeparvovec-xioi). The information below and on the following pages will help you learn more about the disease and the treatment my child received.

## Facts about SMA

**Spinal muscular atrophy (SMA) is a rare genetic disease and, if diagnosed early, can be treated quickly to stop the progression of the disease.**

### How SMA is inherited

SMA is an autosomal recessive disorder. This means that in order to have SMA, a person must have 2 copies of a nonworking *survival motor neuron 1 (SMN1)* gene or be missing both copies of the *SMN1* gene.



### The cause of SMA

The genetic root cause of SMA is the *SMN1* gene that is missing or not working properly. When this main gene is missing or not working properly, the body cannot make enough survival motor neuron (SMN) protein, which is needed for motor neuron cell survival. Everyone is born with a certain amount of motor neuron cells, which are responsible for communicating with the muscles and telling them to work properly. Without enough SMN protein, motor neuron cells throughout the body may lose function and die. As a result, patients with SMA experience muscle weakness and may develop difficulty in breathing, swallowing, or speaking.

**About 1 in 50 people in the United States (or 6.6 million\* Americans) is a genetic carrier of SMA, and most don't know it.**

**A carrier is a person who has a mutation in 1 copy of a gene but doesn't have the disease.**

\*Calculations are based on an estimated US population of 330 million.

### Types of SMA

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



**11K**

**SMA affects about 1 in every 11,000 babies born in the US.**

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

## ZOLGENSMA is changing SMA

ZOLGENSMA brings gene therapy to the forefront of SMA treatment. It is a one-time-only 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



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



- Stops the progression of the disease. ZOLGENSMA is not a cure and cannot reverse any damage done before treatment

### 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 a vein. ZOLGENSMA was not evaluated in patients with advanced SMA.

### IMPORTANT SAFETY INFORMATION

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

 **zolgensma**<sup>®</sup>  
(onasemnogene  
abeparvovec-xioi)  
suspension for intravenous infusion

## SMA after treatment with ZOLGENSMA

While ZOLGENSMA® (onasemnogene abeparvovec-xioi) has replaced the function of 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.

- ZOLGENSMA is not a cure. Children treated with ZOLGENSMA may need additional supportive therapies and accommodations 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.



“

**I want people to see Olivia for all the amazing things that she is. I want her to always feel how our family feels, that she's the most beautiful, special little girl in the world.”**

Kirsten, mother of Olivia

Olivia, treated with ZOLGENSMA at ~2½ months old and pictured at 1½ years old, was diagnosed with SMA Type 1.

# Indication and Important Safety Information

## What is ZOLGENSMA?

ZOLGENSMA® (onasemnogene abeparvovec-xioi) 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, 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.

## 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 Novartis Gene Therapies, Inc. at 1-833-828-3947.**

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

