



# Preparing for SMA after treatment

**Learn about additional therapies,  
accommodations, and support  
that may be needed.**

## 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 additional Important Safety Information on [page 7](#) and the accompanying [Full Prescribing Information](#).**

Malachi, treated with ZOLGENSMA at ~4 months old and pictured at 4 years old, was diagnosed with SMA Type 1.



# Moving forward

Connect with other caregivers  
who share your experience.

Now that your child has been treated with ZOLGENSMA it is important to learn how to continue managing your child's spinal muscular atrophy (SMA) and other health needs. ZOLGENSMA is not a cure and cannot reverse damage already caused by SMA before treatment. Your child still has SMA and may continue to show signs and symptoms now or in the future. These may include difficulty swallowing, difficulty breathing, or muscle weakness. **Call your child's doctor if you see these or any other signs or symptoms.**

Children with SMA may experience or exhibit different signs or symptoms. Therapies, accommodations, and support may be needed to help manage your child's SMA and guide his or her ongoing development.

**The information on the following pages provides a brief introduction to the types of care children with SMA may need.**

Infections before or after ZOLGENSMA infusion can lead to more serious complications. Anyone who has close contact with your child should follow good health and hygiene practices. Contact your child's doctor immediately if your child experiences any signs of a possible infection such as coughing, wheezing, sneezing, runny nose, sore throat, or fever.

**Please see additional Important Safety Information on [page 7](#) and the accompanying Full Prescribing Information.**

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abeparvovec-xioi)  
suspension for intravenous infusion



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**Donovan sees a physical therapist once a week. I'm seeing advancement all the time. It's slow and steady. He has to work a little harder at things, but they do come.”**

Laura, Donovan's mother

Donovan, treated with ZOLGENSMA at ~2 months old and pictured at 4 years old, was diagnosed with SMA Type 1.

## Building a health care team

Your child's health care team should be personalized to his or her needs. A neuromuscular specialist or pediatric neurologist will most likely be the one to monitor your child's ongoing health as it relates to SMA and may serve as the coordinator of care. Other members of your child's care team may include:

- **Pulmonologist**, who specializes in lung conditions
- **Gastroenterologist**, who specializes in the digestive system
- **Registered dietitian/nutritionist**, who provides guidance on adequate nutrition to support growth
- **Orthopedist**, who specializes in bone health
- **Physical therapist**, who teaches therapeutic exercise techniques and recommends physical exercises and equipment
- **General pediatrician**, who provides follow-up and routine care

This is not a full list of health care team members your child may need. Team members can change over time and can differ for every child.

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Maisie, treated with ZOLGENSMA at ~20 months old and pictured at 2 years old, was diagnosed with SMA Type 1.

Maisie started another SMA treatment when she was ~6 months old but discontinued after receiving ZOLGENSMA.

## Breathing support

Breathing problems are common in children with SMA and are considered a serious complication, especially in SMA Type 1 and Type 2. SMA affects the muscles used for breathing, which may lead to the need for breathing support, depending on the severity of the disease. A bilevel positive airway pressure (BiPAP) machine is commonly used in children with SMA who experience trouble breathing. This machine can help push air into the lungs to help support breathing. A cough assist machine may also be used to help with airway clearance. A portable suction machine can help remove excess mucus and saliva that may accumulate in a child's mouth. This is not a complete list of breathing support and every child is different, so ask your child's doctor if ongoing respiratory care or equipment is needed.

▶ Maisie's mom Ciji worked closely with her health care team to determine the best breathing equipment for Maisie's needs.

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## Physical support

Depending on your child's SMA development and symptoms, additional therapists or doctors who specialize in conditions that affect bones, joints, and muscles (the musculoskeletal system) may be a part of your care team. These types of complications may be addressed through therapy, bracing, and/or other supportive equipment. Work with your child's doctor or specialist to continuously monitor any additional issues that may arise.

## Wellness visits

Preventive care is one of the best ways to defend against certain illnesses and injuries, especially those unrelated to SMA. That's why it is important to visit your child's general pediatrician to maintain routine checkups and vaccinations. During these appointments, you can also review any additional SMA support your child may need or goals that you are hoping to achieve. Keeping your pediatrician updated will allow him or her to proactively address concerns now or in the future and provide a bigger picture into your child's medical care.



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**Managing Malachi's SMA involves physical and occupational therapy. We're trying to get him stronger. The stronger he gets, the more he can possibly do.”**

Tina, Malachi's mother

Malachi, treated with ZOLGENSMA at ~4 months old and pictured at 4 years old, was diagnosed with SMA Type 1.

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## Nutrition

For children with SMA, good nutrition is important to support growth and motor function, breathing, and even prevent illness. A registered dietitian or nutritionist can help make sure your child's nutritional needs are being met through his or her diet. This can include monitoring weight, fluid intake, and levels of vitamins and nutrients needed for growth and development. Work with your primary care team for referral to a registered dietitian or nutritionist.

## Care for the caregiver

The demands of being a caregiver can feel overwhelming at times and have a harmful effect on everything from your health to your relationships. That's why it's important to take care of yourself and focus on your own health, too. A few ideas that can help include:

- Carving out time for yourself—work in the garden or relax with a book
- Spending time with friends—grab lunch or go to a movie
- Exercising—try yoga or take a brisk walk around the neighborhood
- Speaking to someone—join a support group and meet others who understand what you are going through

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**“Being knowledgeable about SMA care helps us prepare for the future. If you know about the twists and turns your days can take, you can be one step ahead of the game.”**

Natalie, Eli's mother

Eli, treated with ZOLGENSMA at ~4 months old and pictured at 5 years old, was diagnosed with SMA Type 1. Eli has received another treatment for SMA more than 2 years after receiving ZOLGENSMA.

# 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](#).**

