

Taking the next step

Now that your child has been treated with ZOLGENSMA® (onasemnogene abeparvovec-xioi), 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.

Additional therapies, accommodations, and support may be needed to help manage your child's SMA and guide his or her ongoing development. Use the information on the following pages to talk to your doctor about creating a health plan that works best for your child's needs.

"My advice is to never give up. There are things in life that are going to come at you. Just keep moving on, keep doing what's best for your children, and keep being strong."

Brandon, father of Adalyne
who has SMA Type 1

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](#).

A complete healthcare team

Your child's care team should be personalized to his or her needs. A neuromuscular specialist or pediatric neurologist will 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
- **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

Breathing support

Breathing problems are common in children with untreated 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, or 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. Every child is different, so ask your child's doctor if ongoing respiratory care or equipment is needed.

Supportive therapy

Depending on your child's muscle strength, physical therapy may include activities like stretching and exercising. It may also include bracing or positioning equipment, such as thoracic bracing, wheelchairs, car beds, and standers. Talk with your doctor and physical therapist about the equipment your child may benefit from. Other therapies your doctor may recommend include occupational, speech, aquatic (exercises in a pool), and equine (activities with horses).

Wellness visits

Preventive care is one of the best ways to defend against certain illnesses and injuries. It is important to maintain routine checkups and vaccinations for your child. The earlier a health problem is detected, the sooner a treatment can be started.

Nutrition

A 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.

Care for the caregiver

The demands of being a caregiver can feel overwhelming at times and can 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

PREPARING FOR SMA AFTER TREATMENT

Notes/questions for my child's doctor:

If you have any questions, call your Patient Resource Manager. To learn more about advocacy groups that support and educate families living with SMA, visit ZOLGENSMA.com.



Please see the Indication and 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, or AveXis at 833-828-3947.

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



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