

# ZOLGENSMA® (onasemnogene abeparvovec-xioi) Caregiver Preparation Checklist

This checklist is for informational purposes only.  
We recommend that you always follow advice from the patient's doctor on any medical instructions.



## Before treatment day

### Get confirmation

Confirm your child's infusion date, time, and location with your doctor or care team

Check with your child's doctor to confirm that the necessary baseline tests will be completed before treatment day

» By this time, your child's doctor should have performed the following tests: an AAV9 antibody test and baseline liver function,\* creatinine, complete blood count (including hemoglobin and platelet count), and troponin-I tests

Contact your child's doctor immediately if the patient experiences any signs of a possible infection such as coughing, wheezing, sneezing, runny nose, sore throat, or fever

Ask the treatment center how many family members can be with your child on infusion day

### Safeguard against illness and prepare the home

Keep your family safe. Limiting your child's interaction with sick people or large group settings can reduce the risk of illness and infection

Caregivers and close contacts of patients should follow infection prevention practices (e.g., hand hygiene, coughing/sneezing etiquette, limiting potential contacts).

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

\*Liver function assessment includes clinical exam, aspartate aminotransferase [AST], alanine aminotransferase [ALT], total bilirubin, albumin, prothrombin time, partial thromboplastin time [PTT], and international normalized ratio [INR].

### 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 3 and the accompanying Full Prescribing Information.

Infections before or after ZOLGENSMA infusion can lead to more serious complications. If symptoms of infection appear before infusion, you may be asked to postpone treatment until the infection has resolved

Prepare your child's diaper-changing station at home by purchasing disposable sealable bags and using them for 1 month following infusion

### Fill and administer the prescribed corticosteroid and determine vaccine schedule

Fill your child's prescription in advance for the systemic corticosteroid prescribed by your doctor. The pharmacy may not have it in stock and may need to order it

Give your child his or her first dose of the corticosteroid the day before infusion as directed by your child's doctor. Check with your child's doctor on what time your child should take the corticosteroid. Contact the doctor immediately if your child vomits up the corticosteroid

Talk with your child's doctor about the infusion-day dose of the corticosteroid

» Some treatment centers will not allow you to bring your own prescription of the corticosteroid. If you are unable to bring your own prescription, ask who will supply the corticosteroid, who will administer it, and when the administration will occur

Talk to your child's doctor about potential side effects that may occur after treatment, especially what to do if vomiting or fever occur

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



## Before treatment day (continued)

### Getting ready for infusion

Pack a bag so that you and your family have what you need to be comfortable on infusion day, including snacks, drinks, entertainment, and if needed, disposable diapers, bottles, and formula

Bring something familiar and comforting for your child to have during infusion

Bring a notebook and a pen to keep track of questions you may have before, during, and after the infusion.

If you prefer to take notes on a device, make sure it is charged and that you bring the charger with you

Bring any SMA-related equipment you regularly use. Infusion sites may not have the equipment you need



## Treatment day

Before you leave the hospital, ask your child's infusing doctor or care team to fill out the post-appointment plan in your ZOLGENSMA® (onasemnogene abeparvovec-xioi) post-treatment kit. Your doctor or care team should also review this information with you. The kit includes sections for you to write notes and the contact information of your child's healthcare team

- » Ask the infusing doctor who will be monitoring your child's liver function, creatinine, complete blood count (including hemoglobin and platelet count), and troponin-I levels. Also, confirm if you need to come back to the infusion site for monitoring or if it will be conducted at your pediatrician's office
- » Get the infusing doctor's contact information in case another doctor on your child's healthcare team needs to speak with him or her

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

Visit [ZOLGENSMA.com](https://www.zolgensma.com) for additional resources about treatment day and after treatment.



## After treatment day

Your child will continue to take the corticosteroid after the infusion day as prescribed by your child's doctor. Contact your child's doctor immediately if a dose of the corticosteroid is missed or vomited up

Contact your child's doctor immediately if you see any signs of a possible infection such as coughing, wheezing, sneezing, runny nose, sore throat, or fever

Talk with your child's doctor about scheduling follow-up appointments. The doctor will monitor liver function and other markers through blood tests

- » Weekly liver function (clinical exam and blood work) for at least two months during the corticosteroid treatment and taper periods, and then every other week for another month once the corticosteroid taper period has completed
- » Weekly platelet counts for the first month and then every other week for the second and third months until platelet counts return to baseline
- » Weekly troponin-I for the first month and then monthly for the second and third months until troponin-I level returns to baseline

Talk with your child's doctor to decide if adjustments to your child's vaccination schedule are needed to accommodate treatment with the corticosteroid

- » Protection against influenza and respiratory syncytial virus (RSV) is recommended, consult the patient's doctor

Contact your child's doctor immediately if your child's skin and/or whites of the eyes appear yellowish or if your child experiences a decrease in alertness

Decreased blood platelet counts could occur following infusion with ZOLGENSMA. Seek immediate medical attention if your child 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

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 your regular trash

### Preparing for the future with SMA

Continue with or find a neuromuscular clinic that specializes in SMA and can provide consistent care

- » It is important to remember that your child still has SMA and will need a healthcare team of specialists to take care of his or her needs

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

Please see additional Important Safety Information on page 3 and the accompanying Full Prescribing Information.

## 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 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.

### 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 833-828-3947.

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

