

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

A STEP-BY-STEP GUIDE TO ONE-TIME ZOLGENSMA TREATMENT

Once you and your child's doctor have chosen ZOLGENSMA, there are important details that need to be taken care of. We've created this step-by-step guide that goes over each one to help ensure the process goes smoothly.

Indication

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.

Important Safety Information

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.

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



Malachi with his dad, Torence
2 copies of *SMN2* gene
Treated at ~4 months old



Visit [ZOLGENSMA.com](https://www.zolgensma.com)
to learn more



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





Important lab tests

Your child’s doctor will schedule lab tests to help determine eligibility for your child. It’s generally best to have the tests done as soon as you can so that your child can get treated as quickly as possible; work with your doctor to schedule them.

SMA genetic test

This test will be performed to verify your child’s diagnosis. While your child may have received their SMA diagnosis from a newborn screening, insurance companies often require an additional test to confirm the diagnosis

Appointment date: _____ Time: _____ Location: _____

AAV9 antibody test

ZOLGENSMA travels throughout the body using a delivery vehicle made from a virus called adeno-associated virus 9, or AAV9. This type of virus is not known to make people sick.

If your child’s existing level of AAV9 antibodies doesn’t fall within a certain range, you can ask your doctor to monitor and retest. If antibody levels lower, your child may become eligible for treatment

Appointment date: _____ Time: _____ Location: _____

Baseline blood tests

Your child’s doctor will need to establish a starting point so they can monitor closely after treatment with ZOLGENSMA, keeping an eye on things like liver function, creatinine levels, complete blood count (hemoglobin, platelet count, etc.), and troponin-I

Appointment date: _____ Time: _____ Location: _____

The results of these tests may be available in a few days, though sometimes it takes a bit longer. Ask your doctor if you have any questions about these tests, including what these tests are and why your child needs them, and when you may expect to receive results.

Your notes



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



Your child's doctor submits prescription and consent forms

While waiting for the results of your child's lab tests, ask their doctor to:

Submit the ZOLGENSMA Prescription Form

Submit the Patient Consent Form to the OneGene Program®

Once the signed patient consent form and the ZOLGENSMA prescription form are submitted, you'll be enrolled in the OneGene Program and you will meet your Family Ambassador who will be your main, dedicated point of contact. In addition, you will also have the support of the Case Coordinator, who will work closely with your doctor, Family Ambassador, and you to help with the process for your child to receive ZOLGENSMA.

OneGene Program team members are there for additional support, and do not take the place of the medical expertise your doctor provides. For specific medical questions and guidance, please check with your doctor to receive the most accurate and appropriate information

[Learn more about the OneGene Program and how the support team can help you.](#)

Family Ambassador:

Name: _____

Phone number: _____

Email: _____

Your notes

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





Working with your insurance

Your next step is to get insurance approval. Your child’s doctor’s office will complete and submit prior authorizations and fulfill any appeal requirements.

If you’re enrolled in the OneGene Program®, your dedicated OneGene Team Members are here when you need them:

Family Ambassador:

A Family Ambassador will be your main, point of contact who can answer questions you have related to SMA, ZOLGENSMA, and health insurance coverage. Your Family Ambassador is there to provide support throughout your child’s treatment journey.

Case Coordinator:

A Case Coordinator can discuss financial assistance options with you based on your child's eligibility, answer insurance questions and provide appropriate resources to your doctor when needed, and track ZOLGENSMA treatment from the moment the prescription is written until it arrives at the doctor's office.

Be sure to:

Work with your child’s doctor to help make sure all lab tests are completed

Notify your child’s doctor if additional information is needed

Give your OneGene Program team your child’s coverage information

Ask your OneGene Program team if financial assistance programs are available to you

Have questions? Reach out to your OneGene Program Case Coordinator at **855-441-GENE** (4363)—they’re here to help!

[Learn more about insurance coverage for ZOLGENSMA.](#)

Your notes

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





Preparing for treatment (page 1 of 2)

Here’s what you can do in advance to help make sure treatment day goes smoothly.

Ask when your doctor will weigh your child. This is very important because your child’s weight determines dosage

Date: _____ **Weight:** _____

Confirm the treatment date, time, and location with your child’s doctor or care team before the infusion. And ask about your treatment center’s policy regarding what or who you can bring (you may want to have a family member or a friend accompany you)

Appointment date: _____ **Time:** _____ **Location:** _____

Check with your child’s doctor to **ask if any additional tests are needed before treatment day**, and review the baseline lab work. It’s best to have these tests done as soon as you can so that your child can get treated promptly.

If your child’s doctor has asked for additional lab work, you may want to write down the test name and your appointment details here:

Additional tests (if needed): _____

Watch for signs of infection and **notify your doctor right away** if your child begins coughing, wheezing, sneezing, has a runny nose or sore throat, or develops a fever

Limit contact with others to reduce the risk of illness. Practice good hygiene like coughing or sneezing into a tissue, and washing hands with soap and water for at least 20 seconds

Your notes



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



Purchase disposable, sealable bags for your child’s dirty diapers. You should use them for one month after the infusion

Ask your child’s doctor if you have any specific, unanswered questions, such as information about:

- The infusion
- Post-treatment follow-up and monitoring
- The schedule for the day
- Whether additional baseline blood tests are needed

Pack a bag with everything you and your child may need to be comfortable on treatment day. (Treatment centers may not have the refreshments and equipment you’ll need)

Fill the prescription for the oral corticosteroid as soon as you get it. (Your pharmacy may have to order it)

Give your child the first dose of the prescribed oral corticosteroid the day before the infusion.

Your child’s doctor will discuss this in more detail, including the time of day to take the medication, what protocol to follow, and what to do if your child refuses the dose or vomits it up

Time of day: _____ Your doctor’s directions: _____



You’ll find that your Family Ambassador can be a great source of support and reassurance. They will help you throughout treatment day and be there with reminders any time you might need during the process.

Your notes



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



Preparing for treatment with ZOLGENSMA

TREATMENT DAY!

Now that you have the prep work behind you, you're all set for treatment day, and you can concentrate on being there for your child. And as always, your Family Ambassador is there to support you and your family.

Things to know before arriving for treatment:

Confirm the logistics for the big day including: arrival time, where in the hospital the infusion will take place, and who to ask for when you arrive

Arrival time: _____ **Location in hospital:** _____

Contact name: _____ **Contact phone:** _____

Give your child the second corticosteroid dose before their ZOLGENSMA infusion. Confirm with your doctor whether you should administer this dose before arriving at the hospital

Time of second dose: _____

Expect the infusion to take about 60 minutes. You will be asked to remain at the treatment center for a period of time after the treatment; ask your child's doctor in advance how long you'll be required to stay

Wait time after infusion: _____

Your notes

In hospitals, babies are often administered medications and/or fluids through an intravenous infusion, also called an IV. This means that a catheter (a kind of needle) is placed in a vein, usually a vein in the child's arm or leg. ZOLGENSMA is administered in a similar way—through a catheter using a syringe pump in which the medication is given slowly, over 60 minutes.

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





Immediately after the infusion

Before you leave the infusion center:

Talk to your child’s doctor about what to expect after treatment

Confirm follow-up appointments with your child’s doctor

For the first two months (or longer), your doctor will need to monitor your child’s liver function* with weekly blood tests and clinical exams. After that, when your child stops taking the corticosteroid, your doctor will continue to monitor your child every other week for at least one month.

Your child’s doctor will also run blood tests to keep an eye on platelet counts and troponin-I levels. Use the space below to help you keep track of appointments

	Date	Time	Location
Week 1			
Week 2			
Week 3			
Week 4			
Week 5			
Week 6			

	Date	Time	Location
Week 7			
Week 8			
Week 9			
Week 10			
Week 11			
Week 12†			

*Your child’s liver function will be monitored weekly for **two months or longer**, during the corticosteroid treatment and as the dose is reduced. Your child’s liver function will continue to be monitored every other week for **another month** after stopping the corticosteroid.

†Monitoring beyond 12 weeks may be required.

When you get home:

Continue to give your child the corticosteroid as prescribed by their doctor, and notify the doctor if your child misses or vomits a dose

Monitor for side effects. Contact your child’s doctor immediately if you notice:

- Any signs of infection, including coughing, wheezing, sneezing, runny nose, sore throat, or fever
- Vomiting (after receiving the corticosteroid dose). You should also contact your doctor if your child misses a dose
- Yellowish appearance of the skin or whites of the eyes
- A decrease in alertness
- Unexpected bruising or bleeding
- Seizures
- Decreased urine output
- Any other symptoms your child’s doctor may tell you about



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



After treatment with ZOLGENSMA

Your child's ongoing SMA care

Though ZOLGENSMA stops the progression of SMA by replacing the function of the missing or nonworking *SMN1* gene, ZOLGENSMA is not a cure and cannot reverse damage already caused by SMA before treatment. Here are some guidelines for your child's care moving forward:

Complete your child's blood test appointments for at least 3 months after treatment or longer as directed by your child's doctor

Continue giving your child the oral corticosteroid as prescribed by their doctor

Speak with your child's neuromuscular specialist to determine the best care for your child. Your healthcare team may recommend:

- Physical and occupational therapy
- Working with a nutritionist
- Consulting with a:
 - Pulmonologist (who specializes in the lung)
 - Gastroenterologist (who specializes in the digestive system)
 - Orthopedist (who specializes in bone health)

Ask if your child needs additional support such as:

- A breathing support machine—breathing problems are common in children with SMA and are considered a serious complication
- Physical bracing and other supportive equipment
- Additional wellness visits

Maintain routine checkups and vaccinations and review any additional SMA support and goals

Care for yourself. Looking out for yourself will help you take care of your child. There are many demands on parents and families, and it can affect everything from your health to your relationships

Your notes



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



Your contacts and addresses

Keep contact information all in one place. Add your child's doctors, nurses, testing lab, treatment center, Family Ambassador, etc.

Name _____

Phone _____

Email _____

Address _____

Fax _____

Name _____

Phone _____

Email _____

Address _____

Fax _____

Name _____

Phone _____

Email _____

Address _____

Fax _____

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Indication and Important Safety Information

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- 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.
- There is a theoretical risk of tumor development with gene therapies such as ZOLGENSMA. Contact the patient's doctor and Novartis Gene Therapies, Inc. (1-833-828-3947) if a tumor develops.

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

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



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suspension for intravenous infusion

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Complete
Lab Tests

Submit
Forms

Insurance
Approval

Before
Treatment

**TREATMENT
DAY**

After
Treatment

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