



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 item to help ensure the process goes smoothly.



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

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 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.



Please see additional Important Safety Information on [page 12](#) and the accompanying [Full Prescribing Information](#).

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



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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, and complete blood count (hemoglobin, platelet count, etc)

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 the Indication and Important Safety Information on [page 12](#) and the accompanying [Full Prescribing Information](#).





Getting ZOLGENSMA

Your child's doctor submits Prescription and Start Form

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

☐

Submit the ZOLGENSMA Prescription Form

☐

Submit the Start Form to Novartis Patient Support™

Once the signed Start Form and the ZOLGENSMA Prescription Form are submitted, you'll be enrolled in Novartis Patient Support, and you will meet your Case Coordinator who will be your main, dedicated point of contact. Case Coordinators can provide insurance support, financial support, and ongoing support for eligible patients prescribed ZOLGENSMA.

Dedicated assistance from Novartis Patient Support and educational resources help patients get started on treatment and support them along the way. Novartis Patient Support is not a clinical service and does not replace guidance from health care providers. Our goal is to help patients feel informed about their treatment from day one.

[Learn more about Novartis Patient Support and how the support team can help you.](#)

Case Coordinator:

Name: _____

Phone number: _____

Email: _____

Your notes

Please see the Indication and Important Safety Information on [page 12](#) and the accompanying [Full Prescribing Information](#).

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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 Novartis Patient Support, your dedicated Case Coordinator is here when you need them:

Case Coordinator:

Case Coordinators can provide insurance support, financial support, and ongoing support for eligible patients prescribed ZOLGENSMA, including

- Answering questions related to SMA and ZOLGENSMA
- Explaining the steps before and after treatment with ZOLGENSMA
- Helping navigate the insurance and reimbursement process, including benefits verification and prior authorization and appeals support
- Identifying financial support options and eligibility
- Tracking the ZOLGENSMA treatment from prescription to delivery to the site of administration

Be sure to:

☐

Work with your child's doctor to help ensure all lab tests are completed

☐

Notify your child's doctor if additional information is needed

☐

Give your Novartis Patient Support team your child's coverage information

☐

Ask your Novartis Patient Support team if financial assistance programs are available to you

Have questions? Reach out to your Novartis Patient Support Case Coordinator at **855-441-GENE** (4363)—they're here to help!

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

Your notes

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Here's what you can do in advance to help ensure 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

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Preparing for treatment (page 2 of 2)

☐ **Purchase** disposable, sealable bags for your child's dirty diapers. You should use them for 1 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: _____



Your notes

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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 Case Coordinator 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: _____

☐ **Watch** for infusion-related reactions that may occur during and after infusion

Your notes

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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.

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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 2 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 1 month.

Your child's doctor will also run blood tests to keep an eye on platelet counts. They might also consider a cardiac evaluation and suggest consulting a cardiologist as needed. 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 **2 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 your child. Contact your child's doctor immediately if you notice:

- Any signs of an infusion-related reaction, including rash, hives, vomiting, shortness of breath, respiratory symptoms and/or alterations in heart rate and blood pressure
- Any signs of infection, including coughing, wheezing, sneezing, runny nose, sore throat, or fever
- 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

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After treatment with ZOLGENSMA

Your child's ongoing SMA care

ZOLGENSMA is the only treatment that replaces the *SMN1* gene to stop the progression of SMA with just one dose. 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 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

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Your contacts and addresses

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

Name	_____
Phone	_____
Email	_____
Address	_____
Fax	_____

Name	_____
Phone	_____
Email	_____
Address	_____
Fax	_____

Name	_____
Phone	_____
Email	_____
Address	_____
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Indication and Important Safety Information

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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.

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

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



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