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

A CARE PLAN FOR YOUR CHILD AFTER TREATMENT

What to watch for after your child's infusion with ZOLGENSMA® (onasemnogene abeparvovec-xioi)

- Call your child's doctor with any non–life-threatening questions or concerns. Call 9-1-1 with any life-threatening concerns.
- ZOLGENSMA can increase liver enzyme levels and cause acute serious liver injury or acute liver failure which could result in death.
  - To manage potential elevated liver enzymes, your child needs to be treated with an oral corticosteroid before and after infusion with ZOLGENSMA. Talk to your child's doctor about the recommended dose and potential side effects.
- The corticosteroid (equivalent to oral prednisolone 1 mg/kg/day) should begin the day before infusion with ZOLGENSMA and continue daily for a total of 30 days as your child's doctor monitors their liver function. After 30 days, your child will continue taking the corticosteroid but it may be a different dose.
  - The doctor will monitor your child's liver function after ZOLGENSMA treatment through blood tests and clinical exams and determine when to begin gradually lowering the corticosteroid dose and eventually stop the corticosteroid. The suggested period of lowering the dose (taper period) is no less than 28 days. Your child's doctor will monitor their liver function weekly during the corticosteroid course and taper period (at least 2 months), and then every other week for at least 1 month after your child stops the corticosteroid.
  - The specific corticosteroid treatment course for each patient will be determined by the treating doctor. The treatment course is based on several clinical factors and the judgment of the doctor. Discuss specific treatment recommendations with your child's doctor.
  - Do not abruptly stop using the corticosteroid.
  - Contact your child's doctor immediately if your child's skin and/or whites of the eyes appear yellowish, if your child misses a dose of corticosteroid or vomits it up, or if your child experiences a decrease in alertness.
- Decreased platelet counts could occur following infusion with ZOLGENSMA. Your child's doctor will check your child's platelet counts weekly for the first month. This will continue every other week for the second and third months or longer until the counts return to their baseline levels (what they were before treatment). You should seek immediate medical attention if your child experiences unexpected bleeding or bruising.
- Thrombotic microangiopathy (TMA) has been reported to occur, generally within the first two weeks after ZOLGENSMA infusion. Seek immediate medical attention if your child experiences any signs or symptoms of TMA, such as unexpected bruising or bleeding, seizures, or if urinating less than before.
- 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.
- Increases in cardiac troponin-I levels could occur following ZOLGENSMA infusion. The doctor will monitor your child's troponin-I level weekly for the first month and then monthly for the second and third months or longer until it returns to its baseline level.
- See your child's follow-up doctor for scheduled visits, which may be weekly and may include laboratory monitoring.
- Infections before or after ZOLGENSMA infusion can lead to more serious complications. 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 the Indication and Important Safety Information on page 5 and the accompanying Full Prescribing Information.

A CARE PLAN FOR YOUR CHILD AFTER TREATMENT

- Temporarily, small amounts of ZOLGENSMA® (onasemnogene abeparvovec-xioi) may be found in your child's stool. You should 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.
- Talk with your child'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 your child's doctor.
- Caregivers and close contacts of your child should follow infection prevention practices (e.g., hand hygiene, coughing/sneezing etiquette, limiting potential contacts).

Frequently asked questions

- Who do I see for my child's medical monitoring and lab tests?
- What do I need to watch for after infusion?
- Why do diapers need to be sealed in disposable trash bags?

Make sure to bring a notebook with you every time you go to the doctor's office so you can keep track of your questions and answers.

"It's so important for caregivers to always trust their intuition. If you have any questions, it's critical to talk to your healthcare team. Always reach out."

Samantha, mother of Stella who was diagnosed with SMA Type 1

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Management and monitoring are important elements of the treatment journey

Prior to receiving ZOLGENSMA, your child's doctor will do baseline assessments. After infusion, the doctor will closely monitor your child's liver function, platelet count, and troponin-I levels. It is important to set up a monitoring plan with your child's doctor to have the testing schedule ready. As a reference, take this chart with you to your child's next appointment. The specific monitoring schedule will be determined by your child's doctor.

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.

Baseline assessments prior to infusion												
Liver function,* creatinine, complete blood count (including hemoglobin and platelet count), and troponin-I												
Monitoring after infusion												
Test	**Month 1**				**Month 2**				**Month 3**			
	Wk 1	Wk 2	Wk 3	Wk 4	Wk 5	Wk 6	Wk 7	Wk 8	Wk 9	Wk 10	Wk 11	Wk 12
Platelet count	✓	✓	✓	✓		✓		✓		✓		✓
Troponin-I	✓	✓	✓	✓				✓				✓
Liver function	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.			
	✓	✓	✓	✓	✓	✓	✓	✓		✓		✓

*Liver function assessment includes an exam and blood tests (AST, ALT, total bilirubin, albumin, prothrombin time, PTT, INR).

✓ = monitoring performed. Wk = week.

Your child should be assessed immediately and closely monitored if the liver function tests worsen or if you or the doctor notice signs or symptoms of acute illness, such as vomiting or worsening health.

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Your child's SMA care team

ZOLGENSMA® (onasemnogene abeparvovec-xioi) is not a cure. It cannot reverse damage already caused by spinal muscular atrophy (SMA) before treatment. In addition to seeing your pediatrician for routine care, your child will need to see an SMA expert (a **neuromuscular specialist** or **pediatric neurologist**). This specialist will monitor your child's ongoing health as it relates to SMA and may serve as the point person of your multidisciplinary care team. Other members of your care team will depend on your child's needs but may include the following:

- **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 healthcare team members your child may need. Team members can change over time and can differ for every child.

Use the space below to keep track of the details of your child's SMA care team.

SPECIALTY	NAME	LOCATION	PHONE NUMBER	RESPONSIBILITY

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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.
- 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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