



Key Points:

- On May 24, 2019, the U.S. Food and Drug Administration (FDA) approved a new treatment for spinal muscular atrophy (SMA) called Zolgensma® (onasemnogene abeparvovec-xioi).
- Zolgensma is a gene therapy that replaces the missing or broken SMN1 gene – the gene that causes SMA – with a healthier copy of that gene. This treatment can help to improve muscle and nerve function and quality of life.
- Zolgensma is a one-time treatment given through an intravenous (IV) infusion (an IV needle placed in the vein) in Boston Children's Hospital's Center for Ambulatory Treatment and Clinical Research (CATCR) Outpatient Infusion Clinic. Treatment does not typically require an inpatient hospital stay.
- Zolgensma is for children under the age of 2 who have had a genetic test confirming that they have SMA. Children who have received Nusinersen (Spinraza) may get this treatment if they meet the other health and financial requirements.

What is Zolgensma gene therapy?

- Zolgensma (onasemnogene abeparvovec-xioi) is a new gene therapy used to treat spinal muscular atrophy (SMA). SMA is a rare genetic condition that weakens the muscles throughout the body because cells in the spinal cord and brainstem do not work correctly.
- It is a prescription treatment made available by the pharmaceutical company Novartis.
- The U.S. Food and Drug Administration (FDA) approved this treatment on May 24, 2019.

Who can be treated with Zolgensma?

- Children under the age of 2 who have had a genetic test confirming that they have SMA can be treated with Zolgensma.
- Children who have received Nusinersen (Spinraza) may get this treatment if they meet the other health and financial requirements.

How is Zolgensma given?

- Zolgensma is a one-time infusion treatment given through an intravenous (IV) infusion, which is a needle placed in the vein.
- Zolgensma is given in Boston Children's Outpatient Infusion Clinic (which we call the Center for Ambulatory Treatment and Clinical Research or CAT/CR) by a registered nurse who is trained in this type of treatment for children.

- Zolgensma is given over 60 minutes. After the infusion, your child will stay in the infusion clinic to be watched for at least 3 hours.

How does Zolgensma work?

- The genes in our DNA are the "building blocks" of the cells that make up all of our bodies. Gene therapy fixes genes that are missing or not working the right way.
- Zolgensma replaces the missing or broken SMN1 gene – the gene responsible for causing SMA – with a healthier copy of that gene. How? We'll explain:
 - We must use something to carry the gene into the cell. We call this a *vector*. Viruses are often used as vectors because they are very good at getting inside of cells.
 - We use the AAV9 viral vector to transport the healthy SMN gene to replace the missing or broken SMN1 gene.
 - Once replaced, the new SMN1 gene makes SMN protein, which improves children's muscle and nerve function and quality of life.

Will the AAV9 vector make my child sick?

- No. Scientists deactivate (turn off) the part of the virus that causes infection. They keep the part of the virus that's good at getting into the cell. The inactive

virus will stay in the urine (pee), stool (poop), and saliva for a few days after getting the treatment. We call this *viral shedding*.

What are the risks?

The risks of taking Zolgensma are:

- Inflammation (swelling) of the liver or increased risk of injury to the liver. The health care team will closely watch children taking Zolgensma and give corticosteroid medicine before and after treatment to lower this risk.

What are the risks (*continued*)?

- Thrombocytopenia, which is a low number of platelets in the blood
- High level of troponin-I, which is a blood protein found in the heart
- Unable to easily fight off infections due to taking a corticosteroid medicine (which is required for 3 months after Zolgensma)

What should I watch for before and after infusion with Zolgensma?

- **Yellow coloring of the skin or the whites of the eyes**, as this may be a symptom of liver inflammation.
- **Viral respiratory infections:** It's important to watch your child for viral respiratory infections, like a cold or the flu, before or after Zolgensma treatment, as corticosteroids can lower the ability to fight infection.
 - Contact the doctor or nurse right away if your child has signs of a viral respiratory infection, such as coughing, wheezing, sneezing, runny nose, sore throat, or fever.
 - Keep your child away from people who have symptoms of illness, like cold, cough, fever, or flu-like symptoms. If someone in your home gets sick, try to keep that person in a different room from your child.
- **Increased bruising or bleeding:** After Zolgensma treatment, your child's blood platelet count may go down. Call your doctor or nurse right away if you notice that your child has more bruising or bleeding than normal.
- **Breathing issues:** If your child is having signs of breathing problems (like a weak cough, having a hard time breathing, and/or bluish skin color), call 911 or your local emergency services number right away.

What happens after the treatment?

Before you leave the hospital, talk with your health care team about the follow-up care your child needs.

- After the treatment, your child must stay within 1 hour of Boston Children's Main Campus (300 Longwood Avenue, Boston) until they have their labs done the next day. After that, your family must stay close enough to the hospital to be able to return for daily, weekly, and monthly follow up visits and lab testing.
- Your child will have their liver enzyme levels, platelet counts, and troponin-T levels checked often for at least 3 months after treatment.
- Your child will take a corticosteroid by mouth for at least 90 days after getting Zolgensma. This helps to lower the risk of liver inflammation.
- **Contact your doctor right away if your child misses a dose of the corticosteroid.**
- Your health care team will tell you when it is safe to lower your child's corticosteroid dose and when they can stop taking it.
- Your child will be seen at Boston Children's Spinal Muscular Atrophy Program for visits to check on the therapy's progress.

How should I handle my child's bodily waste?

For a short period, small amounts of AAV9 may be found in your child's bodily waste, like urine (pee) and stool (poop). It's important to follow these safety instructions:

- Use good hand hygiene when coming into direct contact with bodily waste for up to 1 month after getting Zolgensma. If you cannot get to soap and water, use an alcohol-based hand rub.
- Seal disposable diapers in trash bags and throw out with your regular trash.

What do I need to know about vaccinations and Zolgensma?

Since your child must take a corticosteroid after treatment, it may affect the vaccines they can get. Talk with your child's health care team to find out which vaccines are safe for them.

How do I know if my health insurance covers Zolgensma?

Because this therapy is so new, it is not yet clear how many health insurance providers cover this therapy.

We encourage you to talk with your health insurance company and care team.

Boston Children's Financial Services staff will guide you through the insurance approval process.

Who will be part of my child's Zolgensma care team?

Your child's care team for this treatment is:

- Infusion nurses
- Neurologists
- Pharmacists
- Financial Services staff

Contact us

Spinal Muscular Atrophy Program

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This Family Education Sheet is in [Arabic](#) and [Spanish](#).