A SPECIAL CHILD IN MY LIFE WAS DIAGNOSED WITH SMA

Maisie, 2 years old
2 copies of SMN2 gene
Treated at 20 months old

Before receiving treatment with ZOLGENSMA, Maisie received another SMA treatment.

SMN2, survival motor neuron 2.

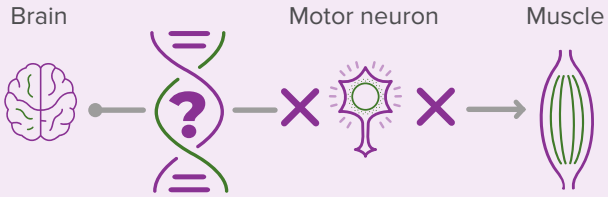
What does that mean?

Spinal muscular atrophy (SMA) is a progressive, rare genetic disease that impacts a child's development. Review the information below and on the following pages to learn more about SMA and how a gene replacement therapy can help.

How does someone get SMA?

SMA is caused by the *survival motor neuron 1* (*SMN1*) gene, which is missing or not working properly. This gene produces the survival motor neuron (SMN) protein that is critical to the function of the nerves that control our muscles. SMA is an autosomal recessive disorder. This means a person must have 2 copies of a nonworking *SMN1* gene or be missing both copies of the *SMN1* gene to have SMA.

Children with SMA



Missing/nonworking *SMN1* gene

Motor neurons can't tell muscles to work

When the *SMN1* gene is missing or not working properly, the body cannot make enough SMN protein. Without enough SMN protein, motor neuron cells throughout the body may lose their ability to function and die. As a result, people with SMA experience **muscle weakness and may develop difficulty moving, breathing, swallowing, or speaking**.

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 5 and the accompanying Full Prescribing Information.

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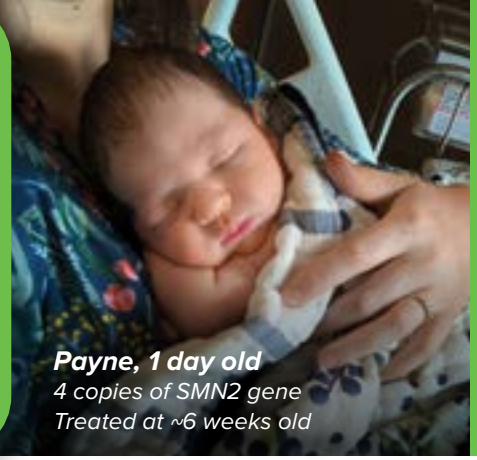
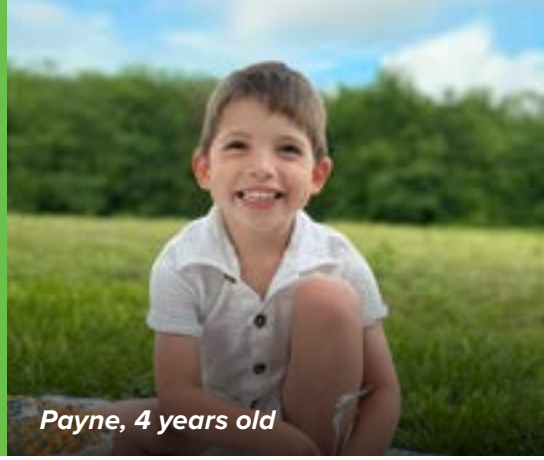
(onasemnogene abeparvovec-xioi)
suspension for intravenous infusion

"Since he was treated... we've had 4 years of joy as we watch him achieve milestones."

—Hannah, Payne's mom



Payne, 1 day old
4 copies of SMN2 gene
Treated at ~6 weeks old



Payne, 4 years old

What about the *SMN2* backup gene?

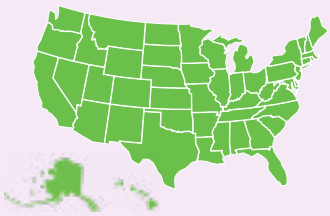
The role of the *SMN2* backup gene

Like many genes, the *SMN1* gene has a backup gene. This backup gene is called the *survival motor neuron 2*, or *SMN2* gene.

For people with spinal muscular atrophy (SMA), the *SMN2* gene is the main source of SMN protein; however, it only makes about 10% of working SMN protein. People can have 1 or more copies of this backup gene and usually, the more copies of the *SMN2* gene a person has, the less severe his or her SMA is.

Even people with several copies of the *SMN2* gene may not produce as much SMN protein as they need, so their motor neuron cells may not work as they should.

As of 2020, SMA experts recommend all babies diagnosed with SMA at birth and who have 2 to 4 copies of *SMN2* be treated for SMA as early as possible in order to stop disease progression—even if they are not showing symptoms.



All 50 states and Washington, DC, screen newborns for SMA at birth

100% of babies born in the United States are screened in this manner. Now that newborn screening is routine, infants are able to receive early treatment, prior to the onset of symptoms.

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.

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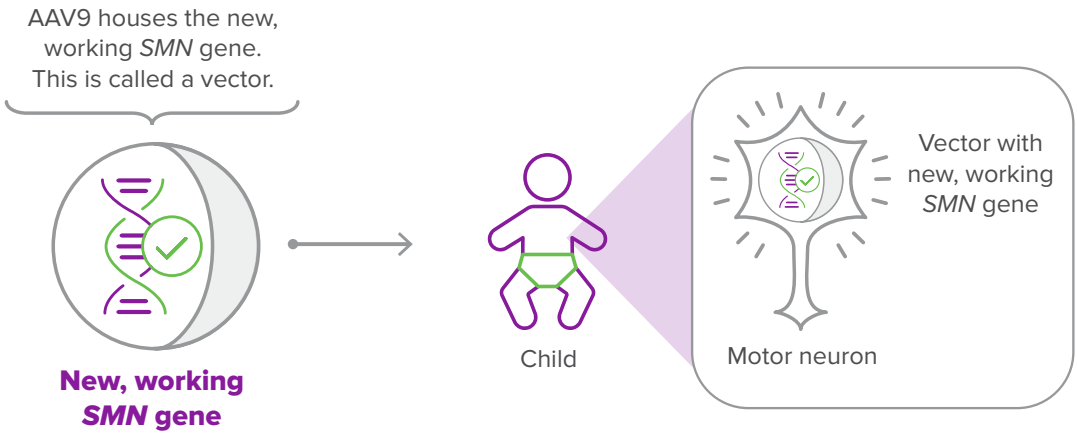
ZOLGENSMA is designed to stop the progression of SMA with one dose

ZOLGENSMA is the only gene replacement therapy for spinal muscular atrophy (SMA) that replaces the function of the missing or nonworking gene.

ZOLGENSMA is made up of a new, working *SMN* gene that is placed inside a delivery vehicle called a vector. The vector that delivers the *SMN* gene is made from a virus called adeno-associated virus serotype 9 (AAV9). This type of virus is not known to make people sick.

By replacing the function of the *SMN1* gene, **ZOLGENSMA enables the production of SMN protein and helps preserve essential muscle function.**



While ZOLGENSMA stops the progression of SMA, it is not a cure and cannot reverse damage already caused before treatment.

ZOLGENSMA is designed to continuously work in the body.

What should I watch for before, during, and after infusion with ZOLGENSMA? (cont)

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.

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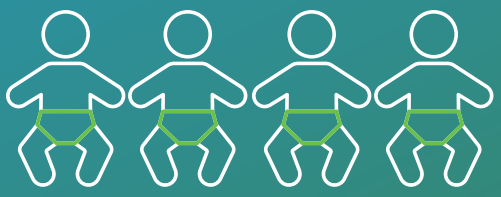
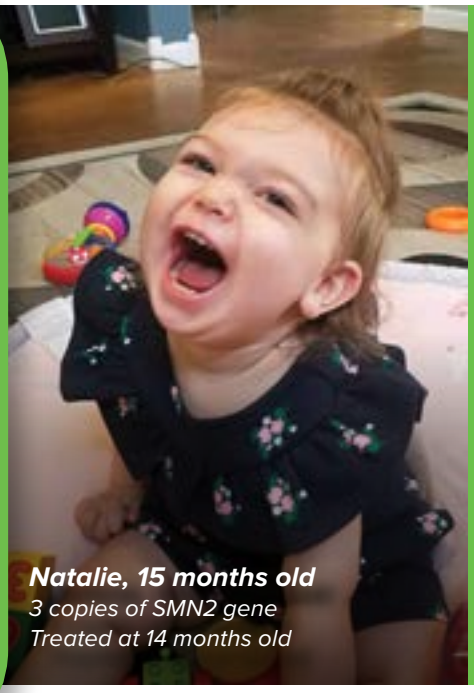
Stopping SMA progression with ZOLGENSMA



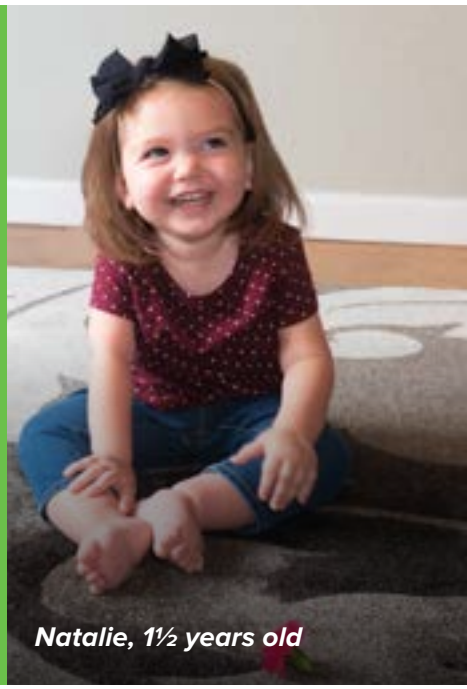
4,000+

children with SMA have been treated with ZOLGENSMA as of August 2024*

SMA, spinal muscular atrophy.



Natalie, 15 months old
3 copies of SMN2 gene
Treated at 14 months old



Natalie, 1½ years old

ZOLGENSMA stops the progression of SMA by helping children meet milestones, like sitting, standing, walking on their own, and more.†

*Globally, including clinical trials, commercially, and through the managed access program.
†Results and outcomes vary among children based on several factors, including how far their SMA symptoms progressed prior to receiving treatment.

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.

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Indication and Important Safety Information

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- 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.

Learn more about treating SMA with ZOLGENSMA.
Visit ZOLGENSMA.com.

NOVARTIS

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