

Children treated with ZOLGENSMA.

## Discussing ZOLGENSMA<sup>®</sup> (onasemnogene abeparvovec-xioi) with your child's doctor

If your child has recently been diagnosed with spinal muscular atrophy (SMA), it's natural to have a lot of feelings and questions. You can help your child by focusing on the next step: getting treatment.

In the most severe types of SMA, **starting treatment as soon as possible is critical for improving outcomes**, such as survival, and reaching and maintaining new developmental motor milestones. Experts also recommend immediate treatment for children with 4 or fewer copies of the *survival motor neuron 2 (SMN2)* gene.

The cause of SMA is a missing or nonworking *survival motor neuron 1 (SMN1)* gene, and only one treatment addresses that genetic cause with just one dose: ZOLGENSMA. This guide can help you and your child's doctor discuss your goals for your child's treatment and decide if ZOLGENSMA is right for them.

Patients will receive an oral corticosteroid and will need blood tests before and after infusion with ZOLGENSMA.

### Indication

#### 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.

### 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 additional Important Safety Information on the last page and the Full Prescribing Information.**

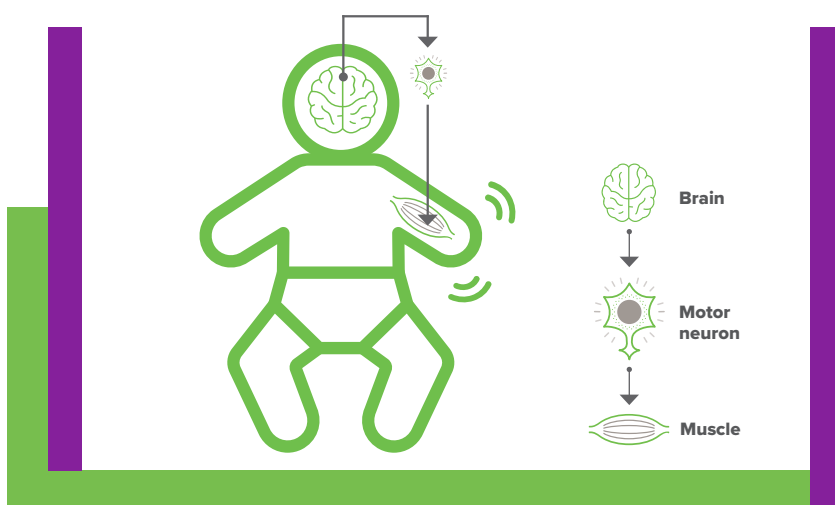
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suspension for intravenous infusion



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## What you need to know about SMA

SMA is a genetic disease caused by a missing or nonworking gene called the **SMN1 gene**. This is the primary gene responsible for producing a protein called **survival motor neuron (SMN) protein**.



**SMN protein is needed to maintain motor neurons, which carry signals from the brain to the muscles.**

Without enough SMN protein, motor neuron cells eventually die and once they are gone, they cannot be brought back. This leads to muscle weakness throughout the body, which affects essential activities, such as walking, eating, and even breathing.

There is a backup gene for *SMN1* called *SMN2*. **SMN2 doesn't produce as much functional SMN protein as SMN1**, but your child may have multiple copies of this backup gene. Generally, the more copies they have, the less severe their SMA. The number of *SMN2* copies is one factor that determines the severity of SMA. Experts recommend immediate treatment for patients with 4 or fewer copies of *SMN2*.

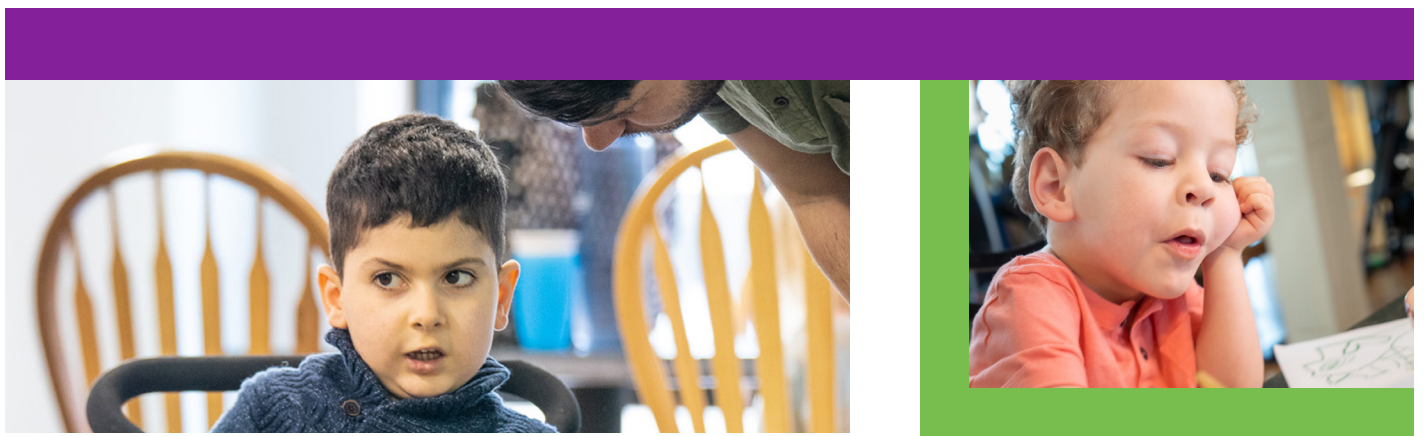
### Important Safety Information

**What should I watch for before and after infusion with ZOLGENSMA® (onasemnogene abeparvovec-xioi)?**

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.

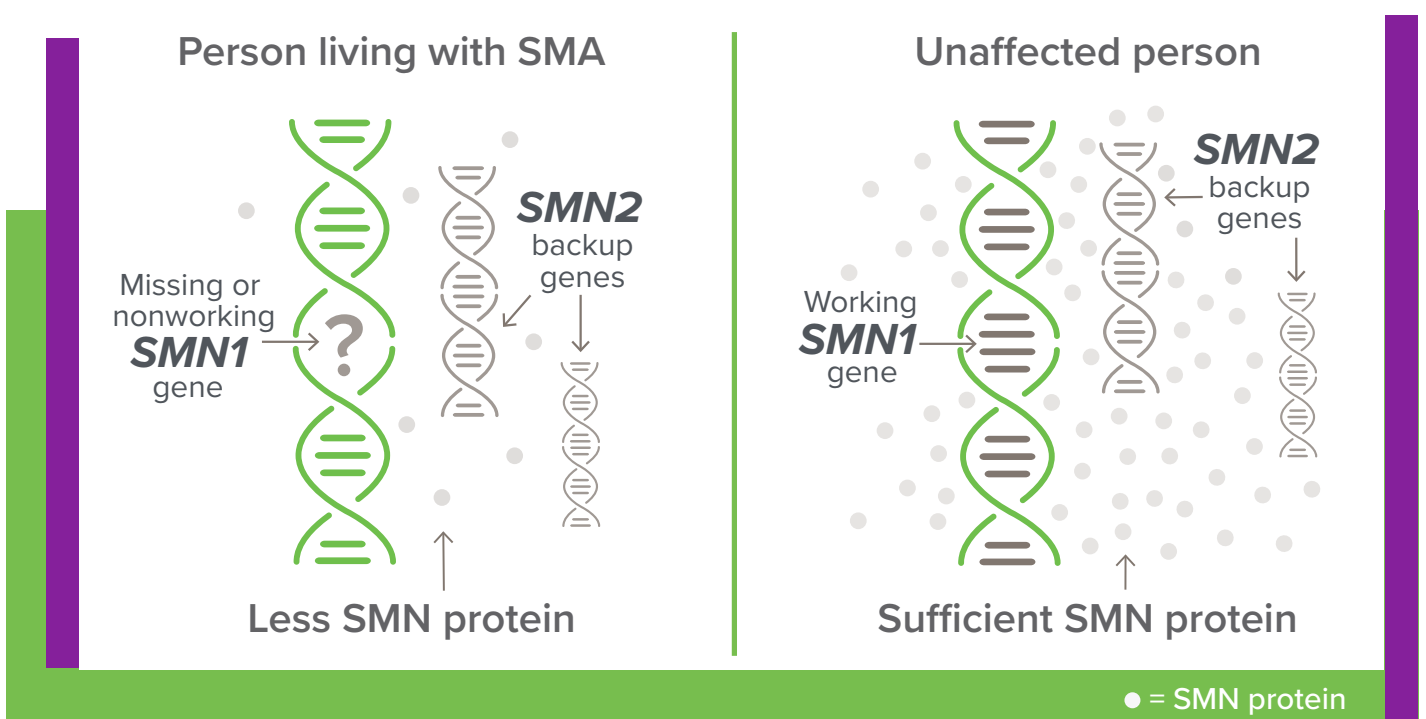
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Even in children with multiple copies of *SMN2*, damage to motor neuron cells still occurs. **The only way to stop further irreversible damage and motor neuron death is by treating SMA as early as possible.**



## How ZOLGENSMA<sup>®</sup> (onasemnogene abeparvovec-xioi) can help

SMA disease-modifying therapies work by increasing the body's supply of functional SMN protein. **Only ZOLGENSMA replaces the function of the missing or nonworking *SMN1* gene with one dose.** Patients will receive an oral corticosteroid and will need blood tests before and after infusion with ZOLGENSMA.

## Important Safety Information

**What should I watch for before and after infusion with ZOLGENSMA? (continued)**

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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All types of SMA are the result of a missing or nonworking *SMN1* gene



Type 1: Non-Sitters



Type 2: Sitters



Type 3: Walkers

Classification without SMA treatment  
← Fewer copies of *SMN2* → More copies of *SMN2*

With just one infusion, ZOLGENSMA® (onasemnogene abeparvovec-xioi):

- Delivers a working copy of the *SMN* gene that is designed to continuously produce functional *SMN* protein
- Helps stop further damage to motor neuron cells



**Did you know?**

ZOLGENSMA is a one-time treatment that delivers a new, fully functioning copy of the *SMN* gene. ZOLGENSMA is given as a one-time-only infusion.

ZOLGENSMA isn't a cure for SMA, but it can stop the progression of SMA. Patients will receive an oral corticosteroid and will need blood tests before and after infusion with ZOLGENSMA.

## Important Safety Information

**What should I watch for before and after infusion with ZOLGENSMA? (continued)**

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.

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## Questions to ask your child's doctor

You and your child's neurologist should work together to choose the right treatment for your child. The following questions can help you have a productive conversation about ZOLGENSMA<sup>®</sup> (onasemnogene abeparvovec-xioi).

### Is treatment with ZOLGENSMA right for my child?

- What sort of results have you seen from children treated with ZOLGENSMA?
- What are the potential benefits and potential risks of treatment with ZOLGENSMA?
- Has ZOLGENSMA been shown to work for my child's type of SMA?
- When is the best time to start treatment with ZOLGENSMA?

Use this space to take notes as you talk through these questions with your child's doctor.

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ZOLGENSMA is the only treatment that replaces the function of the missing or nonworking *SMN1* gene with a one-time-only dose.

Patients will receive an oral corticosteroid and will need blood tests before and after infusion with ZOLGENSMA.

### Important Safety Information

#### 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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## What safety considerations should I know about with ZOLGENSMA® (onasemnogene abeparvovec-xioi)?

- What are the potential side effects of treatment?
- What are the potential risks of treatment?
- What can we do to manage side effects and risks?
- Is the viral vector used to deliver ZOLGENSMA safe?
- Does ZOLGENSMA change my child's DNA?
- Are there any long-term complications from treatment with ZOLGENSMA?

Use this space to take notes as you talk through these questions with your child's doctor.



### Did you know?

Over 3500 children have been treated for SMA with ZOLGENSMA.\* See how some of these children achieved outcomes that would have been impossible without treatment at [zolgensma.com/zolgensma-stories](https://zolgensma.com/zolgensma-stories).

\*Globally, including clinical trials, commercially, and through the managed access programs, as of August 2023.

## Important Safety Information

### 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.

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## What steps do we need to take before starting ZOLGENSMA® (onasemnogene abeparvovec-xioi)?

- What tests are needed before my child can receive ZOLGENSMA?
- Is anyone available to help work with my insurance provider to help ensure treatment with ZOLGENSMA is covered?
- How long will it take to begin treatment when/if we decide ZOLGENSMA is right for my child?
- What should I know about the corticosteroid treatment that is started the day before infusion?

Use this space to take notes as you talk through these questions with your child's doctor.

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## Important Safety Information

### 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.

**Please see additional Important Safety Information on the last page and the Full Prescribing Information.**



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## What should I expect on the day of treatment?

- How is ZOLGENSMA® (onasemnogene abeparvovec-xioi) given?
- Where will my child go to receive ZOLGENSMA?
- How long does the ZOLGENSMA infusion take?
- How many times will we need to come in for my child to receive treatment with ZOLGENSMA?
- Will my child require sedation or anesthesia to receive ZOLGENSMA?

Use this space to take notes as you talk through these questions with your child's doctor.

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**Did you know?**

ZOLGENSMA is the only FDA-approved one-time-only treatment for SMA.

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## What happens after my child receives ZOLGENSMA® (onasemnogene abeparvovec-xioi)?

- What monitoring will my child need after receiving ZOLGENSMA?
- How long will my child need to receive a corticosteroid after receiving ZOLGENSMA?
- How long after treatment will we monitor my child?
- How will we know if treatment is working?
- How long do the effects of ZOLGENSMA last?
- What sort of ongoing care will my child need?
- Who will coordinate my child's ongoing care?

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**Did you know?**

You can count on support from the OneGene Program® before, during, and after your child's treatment with ZOLGENSMA.

**To learn more about SMA and ZOLGENSMA, visit [zolgensma.com](https://zolgensma.com)**

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### 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](http://www.fda.gov/medwatch), or Novartis Gene Therapies, Inc. at 1-833-828-3947.**

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

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