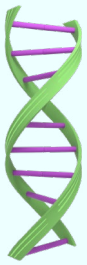
zolgensma®
(onasemnogene abeparvovec-xioi)
suspension for intravenous infusion

Target the genetic root cause of SMA with a one-time dose of ZOLGENSMA

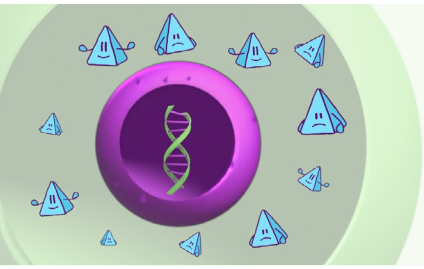
In children with spinal muscular atrophy (SMA), the *SMN1* gene is missing or not working properly. ZOLGENSMA® (onasemnogene abeparvovec-xioi) replaces the function of the *SMN1* gene to help the body continuously produce SMN protein—stopping the progression of SMA with a single infusion. **Here's how it works.**



The *SMN1* gene is the body's main source of SMN protein, and when it is missing or not working properly, it causes SMA

The *SMN1* gene provides instructions for motor neuron cells to make essential SMN protein.

In SMA, the body depends on the *SMN2* backup gene. However, 90% of the SMN protein that the *SMN2* backup gene makes is not fully functional. Usually, the more copies of the *SMN2* gene a person has, the less severe his or her SMA is.

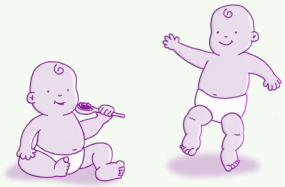


Without enough SMN protein, motor neuron cells stop working and muscles become weak

Motor neuron cells are responsible for all types of muscle movement and when they stop working, they cannot be brought back. This means basic muscle functions become harder to do and can be lost, permanently.

ZOLGENSMA replaces the function of the *SMN1* gene

In a single infusion, ZOLGENSMA delivers a new, working *SMN* gene to the motor neuron cells. A vector acts like a delivery truck to help deliver working *SMN* genes throughout the body.



With a new *SMN* gene, motor neurons can keep working as they should

ZOLGENSMA is designed to provide continuous SMN protein production to stop motor neuron loss and help preserve muscle function.

The sooner treatment is received, the sooner the progression of SMA can be stopped.

Learn about steps to treatment at ZOLGENSMA.com

Indication

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

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 2 and the accompanying Full Prescribing Information.

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.

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 833-828-3947.

Please see the Full Prescribing Information.

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