This document has been approved by Saudi Food and Drug Authority (SFDA)
Important Safety Information

A caregiver's guide to Zolgensma®▼ (onasemnogene abeparvovec) treatment

Your child's doctor has given you this brochure because your child has been prescribed Zolgensma® (onasemnogene abeparvovec) (▼).
This brochure aims to provide practical information to support discussions with your doctor.

Zolgensma (onasemnogene abeparvovec) is subject to additional monitoring to help quickly identify new side effects. If your child experiences any side effects, talk to your child's doctor, nurse or pharmacist/other healthcare professional.
This includes any possible side effects not listed in this guide

If you have any questions or concerns about Zolgensma (onasemnogene abeparvovec) speak with your child's doctor, nurse or pharmacist/other healthcare professional

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Zolgensma Caregiver Information Guide
v1.0 Nov-2021 for distribution in MAP/EAR

Dear parent, carer or family member

Thank you for taking the time to read this guide. You have been given this guide as your child has been diagnosed with spinal muscular atrophy (SMA) and has been prescribed Zolgensma (onasemnogene abeparvovec) by the doctor

You may need to refer to this information again, so please keep a copy of this guide

This guide has been developed to provide useful information on:

- **Zolgensma (onasemnogene abeparvovec) and how it works**
- **Important safety information and possible side effects of Zolgensma (onasemnogene abeparvovec)**
- **Each step of the Zolgensma (onasemnogene abeparvovec) treatment journey**

If you have any questions or concerns about this medicine or your child's health and wellbeing please speak with your child's doctor, nurse or pharmacist/other healthcare professional

We wish you and your family all the best

Useful contacts

SMA, spinal muscular atrophy.

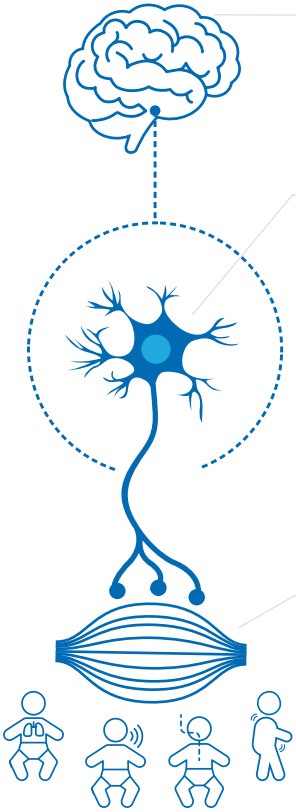
What is in this guide?

SMA, spinal muscular atrophy.

What is SMA?

Spinal muscular atrophy (SMA) is a rare, serious, inherited condition. SMA occurs when there is a missing or abnormal version of a gene needed to make an essential protein called 'survival motor neuron' (SMN). Lack of SMN protein causes nerves that control muscles (motor neurons) to die. This results in muscles becoming weak and wasting away, with eventual loss of movement and difficulty with functions such as breathing, swallowing, feeding and speaking

Unaffected people



Brain

In unaffected people, signals for muscle movement are generated

Motor neurons

These special nerves carry signals from the brain to the muscles to control movement. There are two types of motor neurons: upper motor neurons that carry signals from the brain to the brain stem and spinal cord, and lower motor neurons that carry signals from the upper motor neurons to the muscles

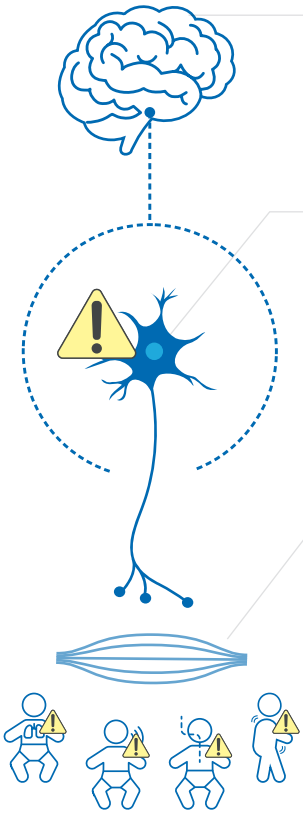
Muscles

Muscles tighten or relax in response to signals carried by the motor neurons. This causes the body to move, and is essential for functions such as breathing, swallowing, feeding and speaking

SMA, spinal muscular atrophy; SMN, survival motor neuron.

Although lower motor neurons are affected by SMA, the ability to think, learn, and build relationships is unchanged

People affected by SMA



Brain

In people with SMA, signals for muscle movement are generated

Dysfunctional motor neurons

People with SMA have a genetic mutation that causes their lower motor neurons to deteriorate and stop working. This means that signals in the brain are not carried from the spinal cord to the muscle

Weakened/wasted muscles

If the muscles no longer receive signals telling them to move they can waste away (atrophy) and become increasingly weaker. This leads to difficulty with movement, breathing, swallowing, feeding and speaking

SMA, spinal muscular atrophy.

What causes SMA?

Unaffected people

Survival motor neuron (SMN) protein

For motor neurons to be able to work properly and survive, the body needs, among other things, a protein called SMN or 'survival motor neuron'. Without SMN protein, motor neurons stop working and die, and muscles become weaker with eventual loss of movement and difficulty with functions such as breathing, swallowing, feeding and speaking

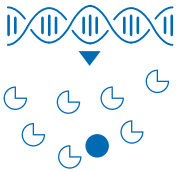
Survival motor neuron *(SMN)* genes

In unaffected people, the body is able to produce the SMN protein from genes called *SMN* genes. There are two types of ***SMN* gene, *SMN1* and *SMN2*:**

Functional SMN protein

Survival motor neuron 1 *(SMN1)*

The *SMN1* gene is the primary gene and produces most of the SMN protein that motor neurons need to work properly



Insufficient amounts of functional SMN protein

Survival motor neuron 2 *(SMN2)*

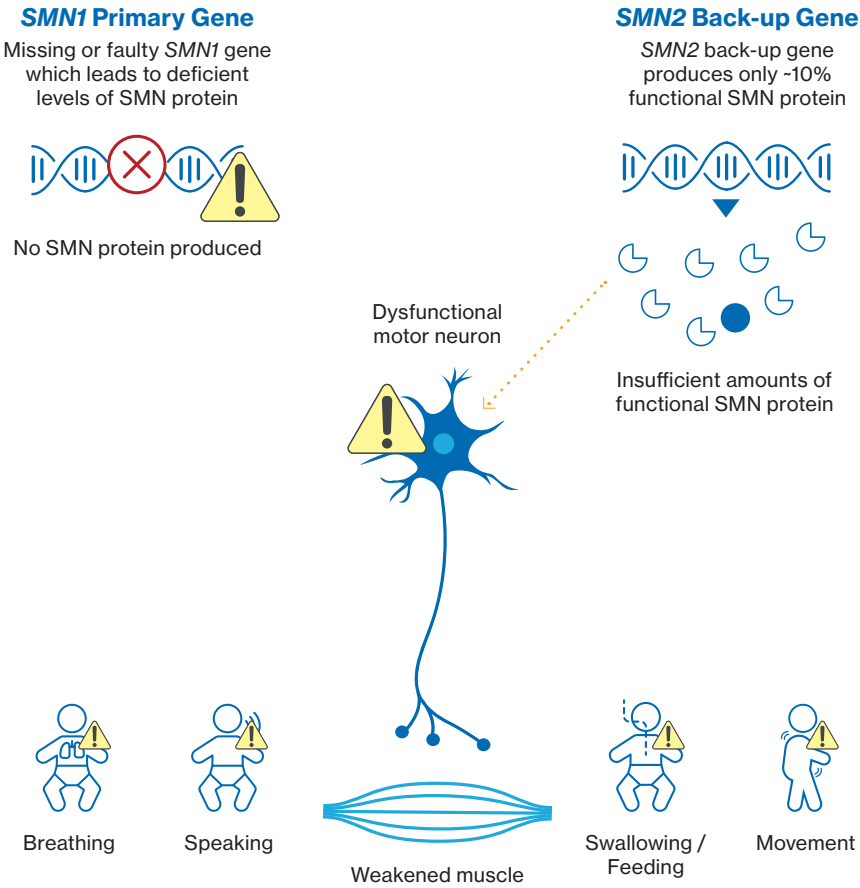
Although the *SMN2* gene also produces SMN protein, it makes several versions of this protein and only small amounts of these work properly ('functional SMN protein'). The *SMN2* gene therefore acts like a 'back-up' to support SMN protein production, but is not able to produce sufficient SMN protein on its own

SMA, spinal muscular atrophy; SMN, survival motor neuron.

People affected by SMA

In people with SMA, the *SMN1* gene is either faulty (mutated) or completely missing

The *SMN2* gene cannot produce enough functional SMN protein to make up for the loss of the *SMN1* gene. As a result, in SMA there is not enough functional SMN protein produced to keep motor neurons healthy and working properly



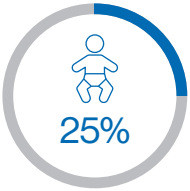
SMA, spinal muscular atrophy; SMN, survival motor neuron.

Who gets SMA?

As SMA is caused by a faulty (mutated) or missing gene, it is considered a genetic disease and is usually inherited

A child inherits two copies of each gene, one from their mother and one from their father. For a child to be born with SMA, they must inherit a faulty gene from both their mother and their father. In SMA, if either copy of the gene (from the mother or from the father) is faulty, the other parent's unaffected gene can make sure enough SMN protein is produced for the motor neurons to work properly. People who have one unaffected copy of the *SMN1* gene and one faulty copy are known as 'carriers' and typically do not show any symptoms. Most carriers have no idea they carry the faulty gene until they have a child born with SMA

Around **1 in 50** people are carriers of SMA, although this will vary slightly depending on your geographical location



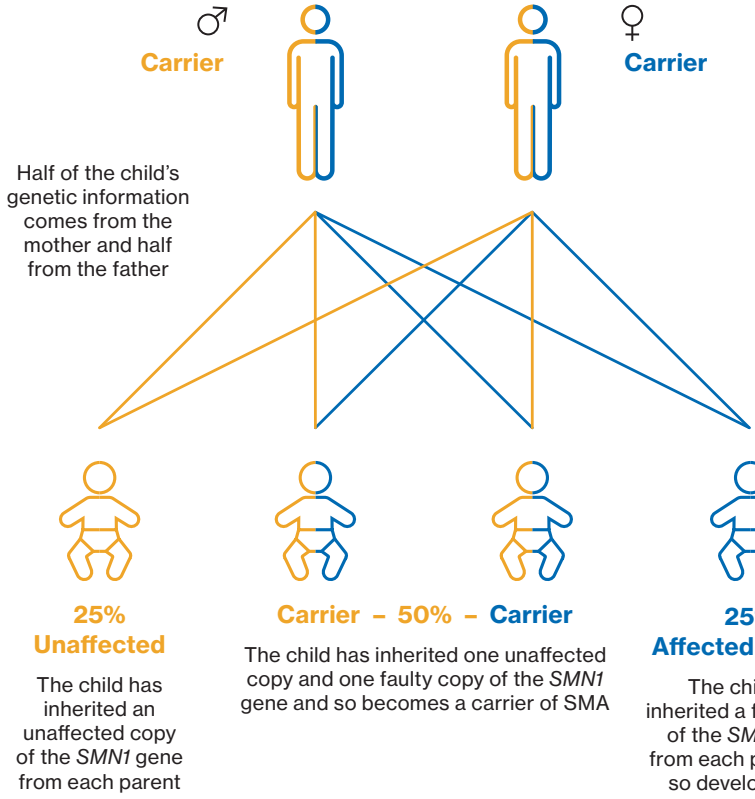
The chance of two carrier parents having a child with SMA is **25%** in each pregnancy

SMA is a rare disease, affecting around **1 in 10,000** newborns, and can impact any race or sex

SMA, spinal muscular atrophy; SMN, survival motor neuron.

How is SMA inherited?

SMA typically happens if a child inherits two copies of the faulty or missing *SMN1* gene, one from their mother and one from their father. This type of inheritance pattern is referred to as 'recessive', and means that, generally, both parents must be carriers for a 25% chance the disease will be inherited



Unaffected Carrier Affected by SMA

All future pregnancies will carry the same 25% chance of having a child affected by SMA, so it is important to discuss your options with your medical team when making decisions on expanding your family

SMA, spinal muscular atrophy; SMN, survival motor neuron.

Severity and symptoms of SMA

You may hear your child's medical team refer to 'motor milestones', which are functional abilities which help to assess SMA and its progression. Motor milestones relevant for children with SMA include their ability to:

Raise their head

Grasp something in their hand

Crawl

Sit

Roll over

Stand and walk

These milestones, together with the age at which symptoms start, are used to help determine the severity of SMA

The earlier symptoms of SMA present, the more severe the disease is likely to be. In this way, the childhood forms of SMA can be deceptive and difficult to diagnose, as children can appear unaffected at birth or even later into childhood

Earlier, more severe → **Later, less severe**

Often in SMA, symptoms present within the first 6 months of life, but in some cases, symptoms may occur before birth, with mothers noticing that their child's movements slow down in the last weeks of pregnancy

For other children, symptoms may not be apparent until much later on in childhood or adolescence. Occasionally symptoms don't appear until adulthood

In the past, children with more severe forms of SMA who did not receive treatment or ventilatory support had a shortened lifespan. However, with early medical intervention and treatment, deterioration can be slowed, with children often able to achieve milestones rarely seen during the natural course of the disease

SMA, spinal muscular atrophy.

SMA is a spectrum disease with a range of symptoms that may vary in severity

	Types of SMA	Symptoms
Birth	**Decreased fetal movement (SMA Type 0)**	In the most severe form of SMA, symptoms usually begin to develop before birth. The child's movements may slow down during pregnancy
≤6 months	**Unable to sit independently (SMA Type 1)**	**Symptoms typically develop by age 6 months and include:** • Poor head control • Weak legs and arms • Difficulty breathing and swallowing • Weak cough and cry
18 months	**Able to sit and may stand, unable to walk independently (SMA Type 2)**	**Symptoms typically appear between 6–18 months of age and include:** • Weak legs and arms • Struggles to sit unsupported • Slow/lost motor milestones • Swallowing and coughing difficulties • Tremor • Pain and difficulty in moving joints • Twisting or curving of the spine • Difficulty breathing in some cases
18 years	**Independent walking (SMA Type 3)**	Symptoms usually develop from early childhood (>18 months) to late adolescence. People with this form of SMA may have poor balance, and have problems with standing up and walking/climbing stairs
	Independent walking into adulthood (SMA Type 4)	In the mildest form of SMA, symptoms don't appear until adulthood. People with this form of SMA typically have mild weakness, without breathing difficulties

SMA, spinal muscular atrophy.

About Zolgensma (onasemnogene abeparvovec)

Zolgensma (onasemnogene abeparvovec) is used to treat babies and young children who have the rare, serious, inherited condition 'spinal muscular atrophy' (SMA)

What is Zolgensma (onasemnogene abeparvovec)?

Zolgensma (onasemnogene abeparvovec) is a type of medicine called a 'gene therapy'. It contains the active ingredient onasemnogene abeparvovec which contains human genetic material

Zolgensma is an adeno-associated virus vector-based gene therapy indicated for the treatment of pediatric patients less than 2 years of age with spinal muscular atrophy (SMA) with bi-allelic mutations in the survival motor neuron 1 (SMN1) gene.

How does Zolgensma (onasemnogene abeparvovec) work?

Zolgensma (onasemnogene abeparvovec) does not change your child's DNA, but replaces the function of the faulty or missing *SMN* gene. Zolgensma (onasemnogene abeparvovec) works by supplying a fully functioning copy of the *SMN* gene which then helps the body produce enough SMN protein. The copy of the *SMN* gene is taken to the cells where it is needed by a gene delivery vehicle known as a 'vector'

The vector that delivers the *SMN* gene is made from a modified virus called adeno-associated virus 9, or AAV9. This type of virus does not cause disease in humans. To make the vector, most of the DNA of the virus is removed and the new *SMN* gene is put inside

AAV, adeno-associated virus; DNA, deoxyribonucleic acid; SMA, spinal muscular atrophy; SMN, survival motor neuron.

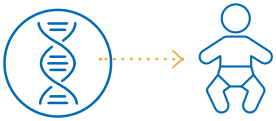
1

Zolgensma (onasemnogene abeparvovec) contains a fully functioning copy of the *SMN* gene

2

In Zolgensma (onasemnogene abeparvovec), the *SMN* gene is placed inside a modified virus (AAV9) that does not cause disease in humans

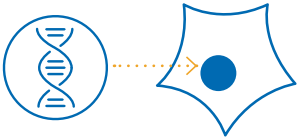
3



Zolgensma (onasemnogene abeparvovec) is given intravenously (into a vein) to your child

4

The modified virus in Zolgensma (onasemnogene abeparvovec) travels through the body and delivers the *SMN* gene to the cells where it is needed



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By supplying a fully functioning copy of the *SMN* gene, Zolgensma (onasemnogene abeparvovec) helps the body produce enough SMN protein

Zolgensma (onasemnogene abeparvovec) in advanced SMA

Zolgensma (onasemnogene abeparvovec) can rescue motor neurons that are still alive and capable of growth, but not dead motor neurons. Children with less severe symptoms of SMA may have enough live motor neurons to benefit from Zolgensma (onasemnogene abeparvovec) treatment. Zolgensma (onasemnogene abeparvovec) may not work as well in children with severe symptoms.

AAV, adeno-associated virus; DNA, deoxyribonucleic acid; SMA, spinal muscular atrophy; SMN, survival motor neuron.

Understanding the risks of Zolgensma (onasemnogene abeparvovec)

Like all medicines, Zolgensma (onasemnogene abeparvovec) can have side effects, although not everybody gets them

IF YOUR CHILD EXPERIENCES ANY SIDE EFFECTS, TALK TO YOUR CHILD'S DOCTOR, NURSE OR PHARMACIST/OTHER HEALTHCARE PROFESSIONAL IMMEDIATELY

Important safety information and when to seek medical attention

To help decide if Zolgensma (onasemnogene abeparvovec) is suitable for your child, your child's doctor will carry out tests for antibodies before treatment.

Liver problems

Tell your child's medical team before your child is given this medicine if your child has, or has had any liver problems. Zolgensma (onasemnogene abeparvovec) can cause an immune response that could lead to an increase in enzymes (proteins found within the body) produced by the liver or injury to the liver. Possible signs you need to look out for after your child is given this medicine include vomiting, jaundice (yellowing of the skin or of the whites of the eyes), or reduced alertness

Abnormal blood clotting

Zolgensma (onasemnogene abeparvovec) may increase the risk of abnormal clotting of blood in small blood vessels (thrombotic microangiopathy). These blood clots could affect your child's kidneys. Tell your doctor immediately if you notice signs and symptoms such as bruising easily, seizures (fits) or decrease in urine output

Low platelet count

Zolgensma (onasemnogene abeparvovec) can lower blood-platelet counts (thrombocytopenia), which can also cause abnormal blood-clotting. Possible signs of a low blood-platelet count you need to look out for after your child is given Zolgensma (onasemnogene abeparvovec) include abnormal bruising or bleeding

Troponin-I

Zolgensma (onasemnogene abeparvovec) can raise levels of a heart protein called troponin-I that may indicate injury to the heart. You need to look out for possible signs of heart problems after your child is given Zolgensma (onasemnogene abeparvovec), such as pale grey or blue skin colour, difficulty in breathing (e.g. rapid breathing, shortness of breath), swelling of the arms and legs or of the belly

Your child will have blood tests to check liver function, kidney function, the amount of blood cells (including red blood cells and platelets) and troponin-I level before treatment with Zolgensma (onasemnogene abeparvovec). In the weeks following treatment, your child will have regular blood tests to check liver function and to monitor for changes in platelets and troponin-I levels. The duration of testing will be decided by your child's doctor

Do not hesitate to talk to your child's medical team if you have any questions

Understanding the risks of Zolgensma (onasemnogene abeparvovec) *(continued)*

Like all medicines, Zolgensma (onasemnogene abeparvovec) can have side effects, although not everybody gets them

IF YOUR CHILD EXPERIENCES ANY SIDE EFFECTS, TALK TO YOUR CHILD'S DOCTOR, NURSE OR PHARMACIST/OTHER HEALTHCARE PROFESSIONAL IMMEDIATELY

Allergies

Your child must not be given Zolgensma (onasemnogene abeparvovec) if they are allergic to any of the ingredients

Other medicines and Zolgensma (onasemnogene abeparvovec)

Zolgensma (onasemnogene abeparvovec) will be given to your child once only. Tell your child's doctor or nurse if your child is taking, has recently taken or might take any other medicines

Vaccinations

As corticosteroids can affect the body's immune system, your child's doctor may decide to delay giving some vaccinations to your child while he/she is receiving corticosteroid treatment. Talk to your child's doctor, nurse or pharmacist/other healthcare professional if you have any questions

Infection

If your child develops an infection (e.g. cold, flu or bronchiolitis) before or after being treated with Zolgensma (onasemnogene abeparvovec) this could possibly lead to other more serious complications

You should tell your child's doctor straight away if you notice your child develops any of the following symptoms:

- Coughing
- Wheezing
- Sneezing
- Runny nose
- Sore throat
- Fever

Corticosteroids

Your child will also be given corticosteroids (prednisolone or equivalent) for a period of time before and after treatment with Zolgensma (onasemnogene abeparvovec). Corticosteroids will help manage any potential increase in liver enzymes that your child could develop following an immune response to Zolgensma (onasemnogene abeparvovec). The dose of corticosteroid your child will receive will be worked out by your child's doctor depending on the child's weight. If you have any questions about corticosteroids, talk to your child's doctor, nurse or pharmacist/ other healthcare professional

Understanding the risks of Zolgensma (onasemnogene abeparvovec) *(continued)*

IN SUMMARY, SEEK URGENT MEDICAL ATTENTION IF YOUR CHILD DEVELOPS ANY OF THE FOLLOWING SIGNS OR SYMPTOMS:

- **Bruising** or **bleeding** for longer than usual if your child has been hurt – these may be signs of a low blood-platelet count (thrombocytopenia)
- **Bruising easily**, **seizures** (fits) or **decrease in urine output** – these may be signs of abnormal clotting of blood in small blood vessels (thrombotic microangiopathy)
- **Vomiting**, **jaundice** (yellowing of the skin or of the whites of the eyes), or **reduced alertness** – these may be signs of possible problems with the liver
- **Pale grey or blue skin color**, **difficulty in breathing** (e.g. rapid breathing, shortness of breath)**, swelling of the arms and legs or of the belly**
- **Coughing**, **wheezing**, **sneezing**, **runny nose**, **sore throat**, or **fever** – these may be signs of infection (e.g. cold, flu or bronchiolitis)

For more details on side effects, please see the Patient Leaflet

Treatment with Zolgensma (onasemnogene abeparvovec)

The steps on the following pages will help you and your family understand what to expect next

1. **Before treatment**
2. **Treatment day**
3. **After treatment**

1. Before treatment

AAV9 antibody test

Antibodies are produced by the body's immune system to help protect it against disease. The presence of certain antibodies, called AAV9 antibodies, can cause your child to have an immune response to Zolgensma (onasemnogene abeparvovec). To help decide if Zolgensma (onasemnogene abeparvovec) is suitable for your child, your child's doctor will carry out tests for antibodies before treatment

Your child may have elevations in AAV9 antibodies, which are often due to the transfer of antibodies from the mother to the baby during pregnancy. These elevations will often decrease over time after birth. If your child has elevations in AAV9 antibodies after their first test, they will be eligible for retesting after a period of time. If you have any questions, please ask your child's doctor

Blood tests

Before Zolgensma (onasemnogene abeparvovec) treatment your child will have blood tests to check and establish baseline levels for:

- Liver function
- Kidney function
- The amount of blood cells (including red blood cells and platelets)
- Troponin-I level

These measurements will help your doctor to monitor your child's levels after Zolgensma (onasemnogene abeparvovec) treatment

AAV, adeno-associated virus.

Corticosteroids

24 hours before being treated with Zolgensma (onasemnogene abeparvovec), your child will be given corticosteroids (prednisolone or equivalent) to manage potential increases in liver enzymes

The dose of corticosteroid your child will receive will be worked out by your child's doctor depending on the child's weight. To make sure that your child does not miss corticosteroid dosing, tell your child's doctor, nurse or pharmacist/other healthcare professional in the event of vomiting before treatment with Zolgensma (onasemnogene abeparvovec)

Infection

It is important to let your child's doctor, nurse or pharmacist/other healthcare professional know straight away if your child develops symptoms of infection. If your child develops symptoms before treatment with Zolgensma (onasemnogene abeparvovec), the infusion may need to be delayed until the infection is resolved. If your child develops symptoms after treatment with Zolgensma (onasemnogene abeparvovec) it may lead to medical complications.
See page 17 for signs of a possible infection

2. Treatment day

Your child's medical team will ensure you know exactly what to expect on the day of treatment and how to prepare

Prednisolone

The first dose of corticosteroids (prednisolone or equivalent) will have been given to your child 24 hours before being treated with Zolgensma (onasemnogene abeparvovec). See page 21 for information on corticosteroid dosing before treatment with Zolgensma (onasemnogene abeparvovec).

On the day of treatment your child will receive their second dose of oral corticosteroids (prednisolone or equivalent) as prescribed. The corticosteroid dosing regimen is important in managing any potential increases in liver enzymes. To make sure that your child does not miss corticosteroid dosing, tell your child's doctor, nurse or pharmacist/other healthcare professional in the event of vomiting after corticosteroid dosing

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Zolgensma (onasemnogene abeparvovec) will be given to your child ONCE only

Zolgensma (onasemnogene abeparvovec) infusion

Zolgensma (onasemnogene abeparvovec) will be given to your child by a single,
intravenous infusion. This involves placing a catheter (plastic tube) into one of your child's veins using a needle. A secondary, back-up catheter will also be inserted in case of any blockage in the primary catheter. The infusion will be given by a doctor or nurse trained in the management of SMA. The infusion will take around 60 minutes

The amount of Zolgensma (onasemnogene abeparvovec) your child will receive will be worked out by your child's doctor depending on the child's weight

SMA, spinal muscular atrophy.

3. After treatment

The duration of hospitalization after treatment with Zolgensma (onasemnogene abeparvovec) will be decided by your child's doctor. If you have any questions, talk to your child's medical team

Possible side effects

Like all medicines, this medicine can have side effects, although not everybody gets them

SEEK URGENT MEDICAL ATTENTION IF YOUR CHILD DEVELOPS ANY OF THE FOLLOWING SERIOUS SIDE EFFECTS

- **Bruising** or **bleeding** for longer than usual if your child has been hurt – these may be signs of a low blood-platelet count (thrombocytopenia)
- **Bruising easily, seizures** (fits) or **decrease in urine output** – these may be signs of abnormal clotting of blood in small blood vessels (thrombotic microangiopathy)
- **Vomiting, jaundice** (yellowing of the skin or of the whites of the eyes), or **reduced alertness** – these may be signs of possible problems with the liver
- **Pale grey or blue skin color, difficulty in breathing** (e.g. rapid breathing, shortness of breath), **swelling of the arms and legs or of the belly**
- **Coughing, wheezing, sneezing, runny nose, sore throat,** or **fever** – these may be signs of infection (e.g. cold, flu or bronchiolitis)

Talk to your child's medical team straight away if your child develops any other side effects. These can include:

Very common (may affect more than 1 in 10 people)

- Increases in liver enzymes seen in blood tests

Common (may affect up to 1 in 10 people)

- Vomiting
- Fever

Reporting of side effects:

If your child experiences any side effects, talk to your child's doctor, nurse or pharmacist/ other healthcare professional immediately

This includes any possible side effects mentioned or not mentioned in this guide

You can also report side effects directly via your national reporting system. By reporting side effects, you can help provide more information on the safety of this medicine

3. After treatment

Corticosteroids

Your child will be given corticosteroid treatment daily for around 2 months after being given Zolgensma (onasemnogene abeparvovec). This period may be prolonged if your child's liver enzymes do not decrease quickly enough, until they decrease to an acceptable level. The dose of corticosteroid given to your child will be slowly reduced during this time until treatment can be fully stopped

Your child's medical team will decide and explain when and how they will stop this treatment for your child. To make sure that your child does not miss corticosteroid dosing, tell your child's doctor, nurse or pharmacist/other healthcare professional in the event of vomiting or any missed doses (for any reason) after treatment with Zolgensma (onasemnogene abeparvovec)

Regular blood tests

Following Zolgensma (onasemnogene abeparvovec) treatment your child will have regular blood tests for:

- Liver function – these tests may take place for at least 3 months after treatment to monitor for increases in liver enzymes
- Blood-platelet count and troponin-I – these will take place for a period of time after treatment to monitor changes in platelets and troponin-I levels

Management of your child's bodily waste

Some of the active substance in Zolgensma (onasemnogene abeparvovec) may be excreted through your child's bodily waste after treatment. You and other people who care for your child should follow good hand-hygiene as outlined below for **at least 1 month** after your child's treatment with Zolgensma (onasemnogene abeparvovec)

Wear protective gloves when coming into direct contact with your child's bodily fluids or waste

Wash hands thoroughly afterwards with soap and warm running water, or an alcohol-based hand sanitizer

Use double plastic bags to dispose of soiled nappies and other waste. Disposable nappies may still be disposed of in household waste

If you have questions on how to handle your child's bodily waste, please talk to your child's doctor, nurse or pharmacist/other healthcare professional

3. After treatment

Supportive care

While Zolgensma (onasemnogene abeparvovec) supplies a fully functioning copy of the *SMN* gene, your child still has SMA

Additional supportive therapies used to care for people with SMA include:

Orthopedic treatments

Physiotherapy

Support with nutrition, eating and drinking

Breathing support, for example with a sleep mask

Prevention of airway infections (e.g. prevention of flu and pneumonia with vaccinations)

Removal of secretions from the airways

Your child's healthcare team will work with you to ensure your child's supportive care needs are managed

SMA, spinal muscular atrophy; SMN, survival motor neuron.

When to seek medical attention

Seek urgent medical attention in any of the following circumstances:

Zolgensma (onasemnogene abeparvovec) can affect the function of the liver and lead to injury of the liver. Possible signs you need to look out for after your child is given this medicine include **vomiting**, **jaundice** (yellowing of the skin or of the whites of the eyes), or **reduced alertness**

Zolgensma (onasemnogene abeparvovec) can lower blood-platelet count (thrombocytopenia). Speak to your doctor if you see signs such as **bruising** or **bleeding** for longer than usual if your child has been hurt

Zolgensma (onasemnogene abeparvovec) can increase the risk of abnormal clotting of blood in small blood vessels (thrombotic microangiopathy). Tell your doctor immediately if you see signs and symptoms such as **bruising easily**, **seizures** (fits) or **decrease in urine output**

Zolgensma (onasemnogene abeparvovec) may cause raised levels of a heart/cardiac-specific protein called 'troponin-I' that may indicate injury to the heart. Speak to your doctor immediately if you see signs and symptoms such as **pale grey or blue skin color, difficulty in breathing**
(e.g. rapid breathing, shortness of breath) or **swelling of the arms and legs or of the belly**

Tell your doctor in the event of **vomiting before or after treatment** with Zolgensma (onasemnogene abeparvovec), to make sure that your child does not miss corticosteroid (prednisolone or equivalent) dosing

Tell your doctor immediately if your child develops signs of infection (e.g. cold, flu or bronchiolitis) before or after being treated with Zolgensma (onasemnogene abeparvovec), as this could possibly lead to other more serious complications. Signs to look out for are **coughing**, **wheezing**, **sneezing**, **runny nose**, **sore throat**, or **fever**

Common words to know

Adeno-associated virus 9 (AAV9)

Adeno-associated virus 9 (AAV9) is a type of virus. AAV9 can be modified so that it does not cause disease in humans. In Zolgensma (onasemnogene abeparvovec), modified AAV9 is the vector used to deliver the fully functioning copy of the SMN gene

Antibodies

Antibodies are produced by the body's immune system to help protect it against disease. Each type of antibody is unique and defends the body against a specific type of disease

Atrophy

Atrophy means to waste away or shrink. For example, a muscle that is wasting away is called an atrophied muscle

Brain stem

The brain stem is a part of the brain that supports critical functions in the body including breathing and sleep. The brain stem joins the spinal cord with the rest of the brain

Corticosteroids

Corticosteroids are a type of medicine which suppress the immune system in order to try to help manage any potential increase in liver enzymes after treatment with Zolgensma (onasemnogene abeparvovec)

Deoxyribonucleic acid (DNA)

DNA, or deoxyribonucleic acid, is the hereditary material in humans and almost all other organisms. Nearly every cell in a person's body has the same DNA

Genes

Sets of instructions that can tell the body how to make proteins. Genes come in pairs; one copy inherited from each parent

Gene therapy

Gene therapy is a way of treating or preventing progression of a disease using genes. There are different types of gene therapy that work in different ways. These include replacing or repairing missing or faulty genes; adding a new gene to help another medicine work better; or stopping the instructions from a gene that is producing too much of a protein that then becomes toxic to the body

Genetic disease

A medical condition caused by a faulty or missing gene or genes. Genetic diseases are inherited. SMA is an example of a genetic disease

Intravenous infusion

An infusion into a vein using a catheter (plastic tube), which is inserted into the vein using a needle

Motor neuron

These special nerves carry signals from the brain to the muscles to control movement

There are two types of motor neurons: upper motor neurons that carry signals from the brain to the brain stem and spinal cord, and lower motor neurons that carry signals from the upper motor neurons to the muscles

Prednisolone

Prednisolone is a type of medicine called a corticosteroid, which helps to manage any potential increase in liver enzymes after treatment with Zolgensma (onasemnogene abeparvovec)

Proteins

Proteins are important molecules involved in nearly every function in the body. Proteins help build the cells of the body, while also helping cells transport and produce important substances, repair and survive

Side effect

A side effect is a secondary, and usually undesirable, effect of a medicine

Spinal muscular atrophy (SMA)

Spinal muscular atrophy (SMA) is a rare disease that causes muscles to gradually become weaker because specialized nerve cells in the body that control muscle movement, called motor neurons, stop working. The motor neurons deteriorate and stop working because they do not have enough SMN protein

SMN1 gene

The SMN1 gene is the primary SMN gene, producing the SMN protein the motor neurons need to work properly. In people with SMA, both copies of this gene are faulty or missing. This means that the cells of the body are not able to produce enough SMN protein

SMN2 gene

The SMN2 gene acts as a ‘back-up’ to support SMN protein production. SMN2 only produces small amounts of functional SMN protein

Survival motor neuron (SMN) protein

SMN protein is vital for motor neurons to work properly and survive. Without enough SMN protein motor neurons deteriorate and stop working. SMN protein is produced by the body from the SMN gene

Useful contacts

This leaflet has been produced by Novartis Gene Therapies. The information provided is for educational purposes only and is not intended to replace discussions with your doctor or care team. Information is related to spinal muscular atrophy and is meant as a general overview

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