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

Your child's doctor has given you this guide because your child has been prescribed Zolgensma®. This guide aims to provide practical information to support discussions with your doctor.

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, speak with your child's doctor, nurse or pharmacist/other healthcare professional.

NOVARTIS

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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 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 focusing on:

- **What is SMA**
- **What is Zolgensma and how does it work**
- **Important safety information and possible side effects of Zolgensma**
- **Each step of the Zolgensma treatment journey**

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

Useful contacts

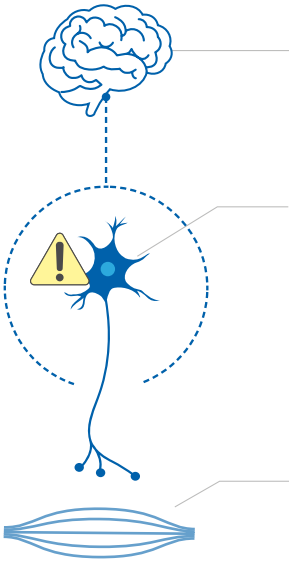
SMA, spinal muscular atrophy.

What is in this guide?

SMA, spinal muscular atrophy.

What is SMA?

Spinal muscular atrophy (SMA) is a rare genetic disease in which nerves are lost causing weakness to the muscles. This occurs because special nerve cells in the body that control muscle movement, called motor neurons, deteriorate and stop working.



Signals come from the brain

In people with SMA, signals for muscle movement are still generated in the brain.

Motor neurons stop working

However, a faulty gene causes special nerves, called motor neurons, that carry signals from the spinal cord to the muscles to stop working. This means that the signals for movement from the brain cannot get to the muscle.

Weakened muscles

If the muscles no longer receive signals telling them to move they become increasingly weaker (atrophied).

In SMA the ability to think, learn, and build relationships is unchanged.

What are the symptoms of SMA?

SMA has a range of symptoms that may vary from person to person and in severity. SMA may affect daily activities such as breathing, eating, sitting, crawling and walking. The earlier symptoms of SMA present, the more severe the disease is likely to be.

SMA, spinal muscular atrophy.

What are the different types of SMA?

At diagnosis, individuals with SMA may be classified into types based on their age of onset and maximum functional ability, or 'motor milestone'. Motor milestones relevant for understanding the type of SMA children may have include their ability to:

Raise their head

Grasp something in their hand

Crawl

Sit

Roll over

Stand and walk

Age at onset	Birth	≤6 months	6–18 months	>18 months	≥18 years
Functional ability	Fetus affected could be before birth	Unable to sit independently	Able to sit, may stand, unable to walk independently	Independent walking	Independent walking into adulthood
Classification	SMA Type 0	SMA Type 1	SMA Type 2	SMA Type 3	SMA Type 4
Severity	Earlier, more severe			Later, less severe	

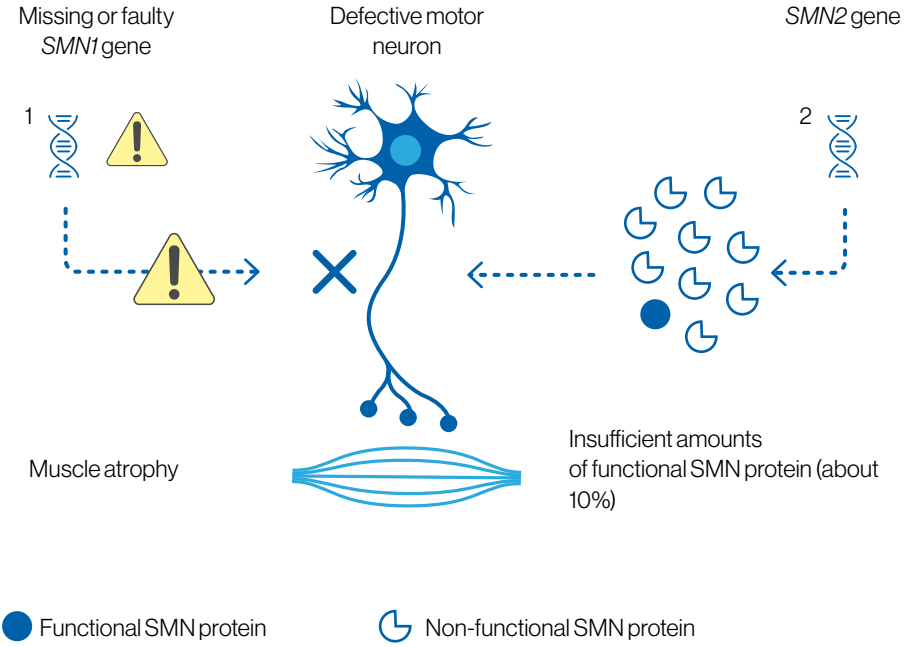
Without intervention or treatment, children with more severe forms of SMA have a shortened lifespan. With early medical intervention and treatment, deterioration can be slowed, with children often able to achieve milestones rarely seen during the natural course of the disease.

SMA, spinal muscular atrophy.

What causes SMA?

A protein called survival motor neuron (SMN) protein is vital for motor neurons to be able to work properly. Without SMN protein, motor neurons in the spinal cord stop working and muscles become weaker. SMN protein is made in the body from the *SMN* gene.

In SMA, the main *SMN* gene known as *SMN1* is faulty or missing. There is a second *SMN* gene known as *SMN2* that acts more like a 'back-up' and only produces small amounts of SMN protein. In SMA, not enough SMN protein is being produced and the motor neurons stop working causing the muscles to become weaker.



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

How do you get SMA?

SMA is typically inherited, although occasionally it is caused by a random error in the *SMN1* gene.

Children inherit two copies of the *SMN1* gene, one copy from each parent. Half of a child's genetic information comes from the mother and half from the father.

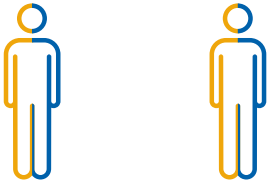
People with one healthy and one faulty *SMN1* gene are known as carriers, they normally show no signs of SMA. If both parents are carriers there is effectively a 25% chance the baby will inherit and develop SMA.

Around **1 in 50** people are carriers of SMA.

SMA affects approximately **1 in 10,000—12,000** live births and can impact any race or sex.

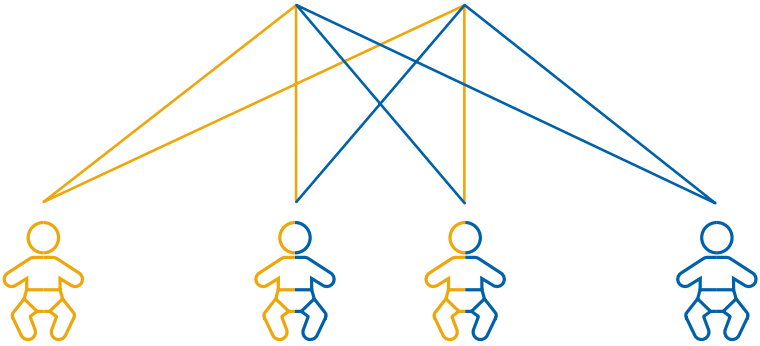
♂ **Carrier**

Holds a defective and a healthy copy of the *SMN1* gene.



♀ **Carrier**

Holds a defective and a healthy copy of the *SMN1* gene.



Healthy

The child has inherited a healthy copy of the *SMN1* gene from each parent and so is unaffected.

Carrier **Carrier**

The child has inherited a healthy copy and a defective copy of the *SMN1* gene and so becomes a carrier of SMA.

Affected by SMA

The child has inherited a defective copy of the *SMN1* gene from each parent and so develops SMA.

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

What is Zolgensma?

Zolgensma is a treatment for babies and young children less than 2 years of age with SMA. Zolgensma is a type of treatment called 'gene therapy'.

Zolgensma is made up of a modified virus, AAV9, which contains a fully functioning copy of the *SMN* gene (the gene that is faulty in SMA). The AAV9 virus in Zolgensma is used to carry the replacement gene into the body and cells where it is needed.

1

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

2

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

3

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

4

By supplying a fully functioning copy of the *SMN* gene, Zolgensma helps the body produce enough SMN protein.

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

Zolgensma in advanced SMA

Zolgensma 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 treatment. Zolgensma may not work as well in children with severe symptoms.

SMA, spinal muscular atrophy.

Understanding the risks of Zolgensma

Like all medicines, Zolgensma 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

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. In some cases, Zolgensma 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.

Injury to the liver can lead to serious outcomes, including liver failure and death. 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. Tell your child's doctor straight away if you notice your child develops any symptoms suggestive of injury to the liver.

Abnormal blood clotting

Zolgensma may increase the risk of abnormal clotting of blood in small blood vessels (thrombotic microangiopathy), generally within the first 2 weeks after Zolgensma treatment. 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.

Extremely close attention should be paid to these signs and symptoms, as abnormal blood clotting (thrombotic microangiopathy) is serious and can be life-threatening if it is not treated.

Low platelet count

Zolgensma can lower blood-platelet counts (thrombocytopenia), generally within the first 2 weeks following Zolgensma treatment. Possible signs of a low blood-platelet count you need to look out for after your child is given Zolgensma include abnormal bruising or bleeding.

Speak to your doctor if you see signs such as bruising or bleeding for longer than usual if your child has been hurt.

Elevated troponin-I

Zolgensma 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, such as pale gray or blue skin color, 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.

For at least 3 months following treatment, your child will have regular blood tests to check liver function and to monitor for changes in platelets and troponin-I levels. Depending on the values and other signs and symptoms, further evaluations may be required. 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 on Zolgensma.

Understanding the risks of Zolgensma *(continued)*

Like all medicines, Zolgensma 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 if they are allergic to any of the ingredients (including onasemnogene abeparvovec, tromethamine, magnesium chloride, sodium chloride, poloxamer 188 and hydrochloric acid).

Corticosteroids

Your child will also be given corticosteroids (prednisolone or equivalent) for a period of time before and after treatment with Zolgensma. The length of time that corticosteroids will be given to your child following Zolgensma treatment will vary depending on liver enzyme values and other signs and symptoms, and will be decided by your child's doctor. This period will be a minimum of 2 months.

Corticosteroids will help manage any potential increase in liver enzymes that your child could develop following an immune response to Zolgensma. The dose of corticosteroid your child will receive will be worked out by your child's doctor depending on your child's weight.

During corticosteroid dosing, your child may face new infections or other usual child illnesses which may require the use of other medicines. Prior to treatment with any other medicines, or if you have any questions about corticosteroids, it is important to consult your child's doctor, nurse or pharmacist/other healthcare professional.

Other medicines and Zolgensma

Zolgensma 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.

Infection

Your child may have a weakened immune system due to corticosteroids, meaning that infections that healthy people can usually fight off may make your child seriously ill. If your child develops an infection (e.g. cold, flu or bronchiolitis) **before** or **after** being treated with Zolgensma this could possibly lead to other more serious complications that may require urgent medical attention.

You should tell your child's doctor straight away if you notice your child develops any signs and symptoms suggestive of infection **before** or **after** Zolgensma treatment, such as:

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

It is important to prevent infections before and after treatment with Zolgensma by avoiding situations that may increase the risk of your child getting infections. You and any other close contacts with your child can help to prevent infection by following good hand hygiene, good coughing/sneezing etiquette, and limiting potential contacts.

Understanding the risks of Zolgensma *(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). Extremely close attention should be paid to these signs and symptoms, as abnormal blood clotting can be life-threatening if it is not treated.
- **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 (including liver failure).
- **Pale gray or blue skin color**, **difficulty in breathing** (e.g. rapid breathing, shortness of breath)**, swelling of the arms and legs or of the belly** – these may be signs of possible problems with the heart.
- **Coughing**, **wheezing**, **sneezing**, **runny nose**, **sore throat**, or **fever** – these may be signs of infection (e.g. cold, flu or bronchiolitis).

Treatment with Zolgensma

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

To help decide if Zolgensma is suitable for your child, your child's doctor will carry out tests for antibodies 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. To help decide if Zolgensma 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. If the baby is new-born, it may be antibodies that have been transferred from the mother to the baby during pregnancy, and 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 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 treatment.

AAV, adeno-associated virus.

1. Before treatment *(continued)*

Corticosteroids

24 hours before being treated with Zolgensma, 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 your 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.

Infection

It is important to let your child's doctor, nurse or pharmacist/other healthcare professional know straight away if your child develops symptoms suggestive of infection **before** or **after** Zolgensma. If your child develops any signs and symptoms suggestive of infection before treatment with Zolgensma, the infusion may need to be delayed until the infection is resolved. If your child develops symptoms after treatment with Zolgensma it may lead to medical complications that may require urgent medical attention.

See page 13 for signs of a possible infection.

Overall health

Before being treated with Zolgensma, it is important that your child's overall health is adequate, otherwise treatment may need to be postponed. This includes being hydrated, having good nutrition and being free of any active infections. If you have any concerns about your child's overall health before treatment with Zolgensma, please speak to your child's doctor, nurse or pharmacist/other healthcare professional.

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. See page 17 for information on corticosteroid dosing before treatment with Zolgensma.

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 and will continue for a period of time after treatment. See page 21 for information on corticosteroid dosing after treatment with Zolgensma.

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.

Zolgensma will be given to your child ONCE only.

Zolgensma infusion

Zolgensma 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 your child will receive will be worked out by your child's doctor depending on your child's weight.

3. After treatment

The duration of hospitalization after treatment with Zolgensma 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.

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). Extremely close attention should be paid to these signs and symptoms, as abnormal blood clotting can be life-threatening if it is not treated.
- **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 (including liver failure).
- **Pale gray or blue skin color, difficulty in breathing** (e.g. rapid breathing, shortness of breath), **swelling of the arms and legs or of the belly** – these may be signs of possible problems with the heart.
- **Coughing, wheezing, sneezing, runny nose, sore throat,** or **fever** – these may be signs of infection (e.g. cold, flu or bronchiolitis).

3. After treatment *(continued)*

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

Common (may affect up to 1 in 10 people)

- Vomiting
- Fever

Reporting of side effects:

If your child experiences any side effects or you are concerned that something might be wrong, 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.

3. After treatment *(continued)*

Corticosteroid dosing after Zolgensma

Your child will be given corticosteroid treatment daily for around 2 months after being given Zolgensma. 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. It is important to keep to the prescribed dosing of corticosteroids. Do not stop corticosteroid treatment prior to discussion with your child's doctor, nurse, or pharmacist/other healthcare professional.

If you have any questions about corticosteroids, talk to your child's doctor, nurse or pharmacist/ other healthcare professional.

3. After treatment *(continued)*

Regular follow-ups

Following Zolgensma treatment, your child will continue to be under the regular supervision of a dedicated healthcare team. Your child will require follow-up visits as necessary, whether these are for general check-ups, if your child is experiencing any side effects, or if you have any concerns or queries.

Appropriate standard of care for patients with SMA, including supportive care, is necessary and will be provided. It is important to discuss with your child's doctor how the healthcare team will continue to support your child.

Regular blood tests

Following Zolgensma treatment your child will have regular blood tests for:

- Liver function – these tests will take place for at least 3 months after treatment to monitor for increases in liver enzymes. If your child's liver function worsens after Zolgensma treatment, or they show any signs of illness, they will be promptly assessed and closely monitored by their doctor.
- 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.

It is important to keep to the scheduled blood tests. Depending on the results of these blood tests, as well as other signs and symptoms, further testing may be required. It is important to closely follow the blood test schedule and to immediately report to your child's medical team all signs and symptoms that your child may develop after they receive their treatment.

3. After treatment *(continued)*

Management of your child's bodily waste

Some of the active substance in Zolgensma 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.

Wear protective gloves when coming into direct contact with your child's bodily fluids (urine) or waste (feces).

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

Use double plastic bags to dispose of soiled diapers and other waste. Disposable diapers 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 *(continued)*

Supportive care

While Zolgensma 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, e.g 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:

In some cases, Zolgensma 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 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 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 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 gray 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, to make sure that your child does not miss corticosteroid (prednisolone or equivalent) dosing.

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

Institution/clinic contact details

Your child will continue to be monitored following Zolgensma infusion by a team of healthcare professionals. If you have any concerns and want to speak to your child's doctor or healthcare team, you can use the below form to note down the contact details of the institution/clinic.

Name:
Role:
Contact number:
Contact e-mail:

Name:
Role:
Contact number:
Contact e-mail:

Name:
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Name:
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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, 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.

Deoxyribonucleic acid (DNA)

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

Genes

Genes are 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.

Platelets

A type of cell that is found in the blood, which is responsible for blood clotting.

Prednisolone

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

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)

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.

Notes

This guide has been produced by Novartis Europharm Limited. 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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zolgensma®
(onasemnogene abeparvovec)

EU RMP v4.0

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