



Dear Parents and Caregivers,

Novartis, the sponsor of this clinical trial COAV101B12301: A clinical trial to learn about the effects of OAV101 in children with spinal muscular atrophy who had not been treated before, would like to thank all the children and their parents for taking part in this trial for the drug OAV101, also called onasemnogene abeparvovec.

You and your child gave up your precious time to be part of our trial. Your child received the trial treatment and had health checks to help researchers learn about the effects for children with spinal muscular atrophy.

Here is a summary of the trial's results. We have provided 3 versions. A version for children from ages 6 months to 9 years, children ages 10 to 17 years, and a version for adults, all of which can be found at www.novctrd.com. Please read the adult version first, for your awareness. Based on your child's age, choose 1 of the 2 children's versions to read with your child. Please contact the research doctor if you have any questions.

Sincerely,

The Novartis Clinical Trial Team

The next page has a Certificate of Participation for children 6 months to 9 years

Thank you!

Thank you for taking part
in this clinical trial!

You helped doctors learn how this
new medicine works for children
with spinal muscular atrophy.

If you have questions about the trial,
ask your parents or another adult.



You are a star!

[Put your child's
photo here]

Name

Date

The next page starts the Plain Language Trial Summary for children 6 months to 9 years

A clinical trial to learn about a new medicine

A story starring you
and other children with
spinal muscular atrophy



**In a clinical trial, people help doctors
to learn about a new medicine**

There are children all around the world who have spinal muscular atrophy, just like you!



What is spinal muscular atrophy, or SMA?
SMA stops your muscles from growing strong. When this happens, it makes it hard to stand up, move, and breathe.





When you were little, you helped doctors learn about a new medicine for SMA.



Doctors wanted to learn if you and other children could move more after getting the new medicine.

**You and 125 other children
with SMA were in this clinical trial.**



**When you went to the doctor, they
checked to see if you could move more
after getting the new medicine, such
as to stand or take a step. The doctors
checked the other children, too.**

Children were able to move more 1 year after getting the new medicine.



The doctors did not find anything new to worry about the safety of the new medicine.



Thank you for taking part in this clinical trial!

You helped doctors learn how
this new medicine works for
children with SMA.
If you have questions about
the trial, ask your parents or
another adult.

You are the star
of this story!

[Put your child's
photo here]

Name: _____

Date: _____

A summary of clinical trial COAV101B12301



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The next page starts the Plain Language Trial Summary for children 10 to 17 years

Read about this clinical trial's results



Hello!

Thank you for being in this clinical trial!

You helped doctors learn about a trial medicine called **OAV101**. The trial medicine is for children and teenagers with **spinal muscular atrophy (SMA)**. We hope you feel proud of your role!



What is a clinical trial and why did this trial happen?

A **clinical trial** is research to learn about a trial medicine. In this trial, doctors wanted to answer these questions about **OAV101** in children and teenagers with **SMA**:

- **Did children's and teenagers' ability to move improve 1 year after getting OAV101?**
- **What medical problems happened to children and teenagers during this trial?**

What is spinal muscular atrophy (SMA)?

SMA is a **genetic condition** that causes muscles to be weak. In **SMA**, the cells that control muscle movement, called nerve cells, die. Since the nerve cells can't tell the muscles to work, the muscles in the arms, legs, chest, face, throat, and tongue become weak. Children and teenagers with **SMA** often have a hard time moving, standing up, and swallowing.



What is a genetic condition?

A **genetic condition** is caused by a problem with a gene. A **gene** is a part of DNA. DNA is like a special code inside cells that tells cells what to do and how to grow. Each gene has a job, like telling a muscle how to move or hair how to grow. In SMA, a problem with the **SMN1 gene** makes nerve cells not work as they should.



What is OAV101?



OAV101 is a trial medicine designed to treat **SMA** by correcting the **SMN1 gene**.



In this trial, children and teenagers got **OAV101** as an injection through the lower back, into the fluid around the spinal cord.

Who was in this trial?



126

children and teenagers were in this trial. Everyone had SMA that had never been treated.

They were

2

to

16

years old



62

boys



64

girls



Where did this trial take place?

The map below shows how many children and teenagers took part in each country.



What happened during this trial?



Children and teenagers got 1 or both of these:

- **OAV101**
- A **sham procedure**



What's a **sham procedure**?

The **sham procedure** was a needle prick in the skin in the lower back one time. The sham procedure did not inject anything into the body.



This trial had 2 parts:

In **Part 1**, doctors used a computer to assign children and teenagers by chance to a treatment group.



Group 1 got OAV101

Group 2 got the sham procedure



Doctors checked children's health for about 1 year.

In **Part 2**, children and teenagers in both groups who continued the trial, got the other treatment:

Group 1 got the sham procedure

Group 2 got OAV101

The children and teenagers and the doctors did not know what treatment group people were assigned to. In each part, children and teenagers got **OAV101** or the **sham procedure** one time.



Doctors checked their health for about 3 months after treatment in Part 2.

What was learned in this trial?



Did children's and teenagers' ability to move improve 1 year after getting **OAV101**?



Doctors used a tool called the **Hammersmith Functional Motor Scale-Expanded (HFMSE)** to measure how much children and teenagers could move. They used the HFMSE to give each child or teenager a movement score before treatment and again 1 year after treatment in Part 1. A **higher score** meant that a child or teenager could move more.

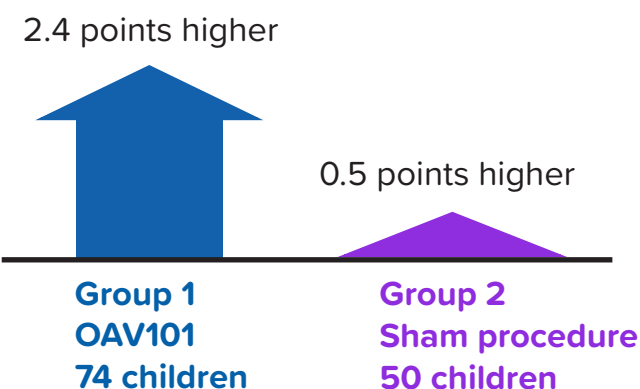


Answer

Children's and teenagers' movements improved 1 year after getting **OAV101** in Part 1 compared to those who got the **sham procedure**.

Change in movement score 1 year after treatment in Part 1

Overall, the change in movement score for children who got **OAV101** was higher than children who got the **sham procedure**.



What was learned from this trial?

What medical problems happened to children and teenagers during this trial?



Doctors checked children and teenagers and wrote down all medical problems they had during the trial, even if they were not caused by **OAV101**.



Most children and teenagers had medical problems during this trial.

In Part 1:

- 38 children and teenagers had serious medical problems.
- 19 children and teenagers had expected medical problems, which were based on past trials of **OAV101**. These included liver or blood clotting problems.
- 32 children and teenagers had medical problems that doctors thought could have been caused by **OAV101**.

Overall in children and teenagers who received **OAV101 in either part:**

- 34 children and teenagers had serious medical problems.
- 21 children and teenagers had expected medical problems, which were based on past trials of **OAV101**. These included liver or blood clotting problems.
- 36 children and teenagers had medical problems that doctors thought could have been caused by **OAV101**.

The most common serious medical problems were:



Infections in the lungs or airways



Throwing up

Other common medical problems were:

- Infection in the nose, throat, or airways
- Fever
- Cough
- Throwing up
- Trouble passing stool (poop)

What else was learned?

Could children and teenagers move their arms and hands more 1 year after getting **OAV101**?



Children and teenagers could move their arms and hands more 1 year after getting **OAV101** in Part 1, compared to those who got the **sham procedure**.

Thank you!

Trials like this cannot happen
without you and others who join.



Doctors need the results from many trials to find new, possible medicines. We learned many things about **OAV101** from this trial.



If you would like to **learn more**, ask a parent or another adult, or read the main summary for this trial at: www.novctrd.com

Search with the trial number
COAV101B12301



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