



Dear Parents and Caregivers,

Novartis, the sponsor of this clinical trial COAV101B12302: A clinical trial to learn more about the safety of OAV101 in children with spinal muscular atrophy (SMA) who stopped other treatments, 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 drug and did health checks to help researchers learn more about the safety and effects for children with SMA.

Here is a summary of the trial 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 about
the safety of this new medicine
for children with SMA.

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 (SMA)



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

There are children all around the world
who have SMA, just like you!



What is SMA?

SMA stops your muscles from growing strong.
When this happens, it makes it hard to stand up
up, move, and breathe.





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



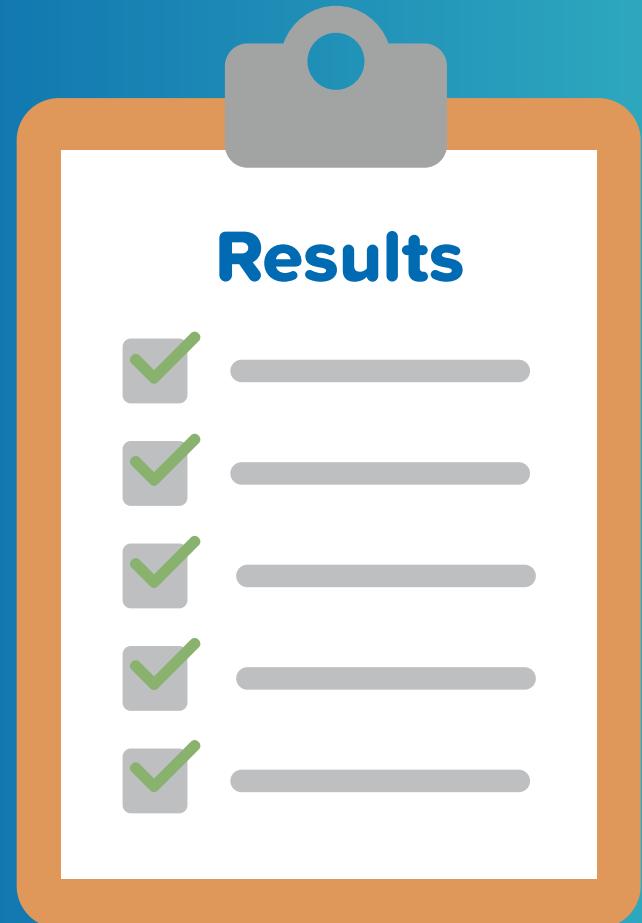
Doctors wanted to learn if the new medicine was safe for children who had stopped taking other medicines for SMA.

You and 26 other children with SMA got the new medicine. All the children were between 2 and 17 years old.



When you visited the doctor, they checked your health.
This helped them learn about the safety of the new medicine for children with SMA.

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



The doctors also wanted to learn if you and other children with SMA could do certain movements, like sit or stand, after getting the new medicine.



The children were able to do the same movements after getting the new medicine as before.

Thank you for taking part in this clinical trial!

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

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You are the star
of this story!

[Put your child's
photo here]

Name: _____

Date: _____

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A summary of clinical trial COAV101B12302



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www.novartis.com/clinicaltrials

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 this question about **OAV101** in children and teenagers with **SMA**:

- **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 die. Since they 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**, there is a problem with the **SMN1 gene**. Because of this problem, nerve cells don't work as they should.



What is OAV101?



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



A **trial medicine** means that doctors are studying it to learn if it works.



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?



27

Children and teenagers were in this trial. Everyone had **SMA** and stopped taking their regular treatments for **SMA** before getting **OAV101** in this trial.



They were
2 to 17
years old



15
boys



12
girls

Where did this trial take place?

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



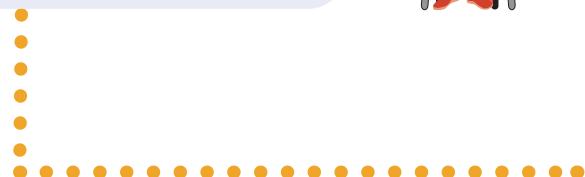
What happened during this trial?



Everyone got **OAV101**.



They got it one time and stayed in the hospital for 2 days or more after getting it.



Doctors checked their health for about 1 year after getting **OAV101**.



At the end of this trial, the children and teenagers were invited to join another trial called **COAV101A12308**. The goal of that trial was to learn about the long-term safety and effects of **OAV101**.

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**.



Answer

All children and teenagers had medical problems during this trial:

- 4 children and teenagers had serious medical problems
- 13 children and teenagers had expected medical problems, which were based on past trials of **OAV101** and included liver or blood clotting problems
- 13 children and teenagers had medical problems that doctors thought could have been caused by **OAV101**, including throwing up, headache, and fever

What was learned from this trial?

The most common serious medical problems were:



Lung infections



Stomach flu

Other common medical problems were:



Common cold



Throwing up



Fever

What else was learned?



Did the children's and teenager's ability to move stay about the same 1 year after getting OAV101?



Answer

Although **SMA** causes the body to lose muscle movement over time, children's and teenager's movements stayed about the same from before treatment to 1 year after getting **OAV101**. This means that children's and teenager's ability to move did not get worse after treatment.



Was day-to-day life easier for the caregivers of the children and teenagers 1 year after getting OAV101?



Answer

Day-to-day life was about the same for the caregivers of the children and teenagers 1 year after getting **OAV101**.

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
COAV101B12302



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