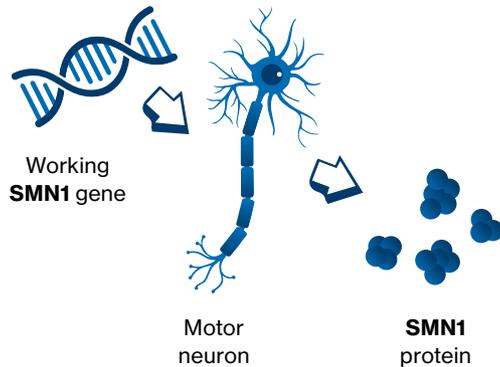


What is the purpose of the STEER study?

The purpose of the STEER Study is to understand if the investigational **gene therapy OAV101** is safe and can provide benefit to children aged 2 to less than 18 years with Type 2 spinal muscular atrophy (SMA).

OAV101 is a gene therapy that contains a working copy of the **SMN1** gene needed by motor neurons to make the SMN protein. The goal is to increase the amount of SMN protein in order to prevent any further loss of motor neurons.



Thank you for considering the STEER study

Clinical studies are types of research studies done to learn more about how the body responds to a certain medicine and whether that medicine works for a specific disease or medical condition.

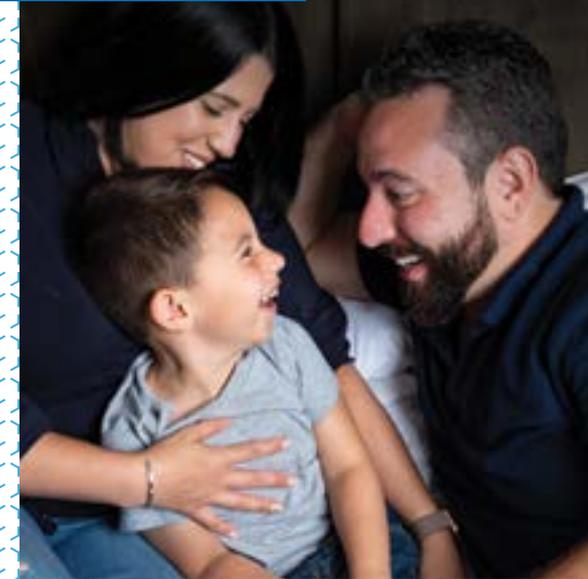
Your child may or may not directly benefit from being in this study, but information learned may help other children with spinal muscular atrophy (SMA) in the future.

Being in a clinical study is voluntary. You and your child can stop participating at any time.

If you are interested in learning more about the STEER Study, contact

at _____

Clinical Trials



Understanding the STEER Study OAV101B12301

For children aged 2 to less than 18 years with Type 2 spinal muscular atrophy (SMA)

Who is the study for?

Children aged 2 to less than 18 years with Type 2 spinal muscular atrophy (SMA) who:

- Are able to sit independently but have never been able to walk independently.
- Began showing symptoms of spinal muscular atrophy (SMA) at 6 months of age or older.
- Have not yet received any treatments that increase SMN.

What happens during the study?

If eligible, your child will be in the study for up to 72 weeks (about 17 months) and have about 29 visits for tests to check on their health. Your child will also have 2 hospital stays to receive treatment.

Screening

Up to 60 days | At least 2 visits

Baseline (study start)

1 day before treatment | 1 visit

Treatment Period 1

Hospital stay

Follow-up Period 1

52 weeks (1 year) | 18 visits

Treatment Period 2

Hospital stay

Follow-up Period 2

12 weeks (3 months) | 8 visits

At the end of the study, your child will have the option to enrol in a long-term follow-up study in which your child's health will be monitored for an additional 15 years.

How is the study treatment given?

- OAV101 is given as a one-time injection into the lower part of the spine. This is called an intrathecal injection.
- Your child will receive the study treatment in the hospital and will stay overnight for a few days.

Treatment Period 1

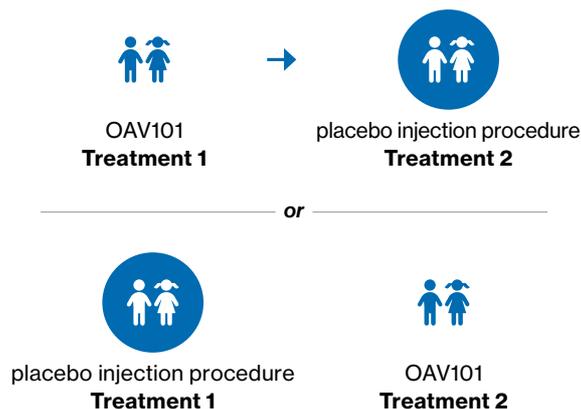
Your child will be assigned by chance to receive either OAV101 or a "placebo injection procedure".

- The placebo injection procedure is a needlestick to the lower back. No medicine is given. The placebo injection procedure is important so the doctors can better understand the effects of OAV101.
- You and the study doctor will not know which group your child is in. This helps to keep the study fair and unbiased.

Treatment Period 2

After completing 1 year of follow-up visits, your child will have a few health checks to make sure he/she is eligible to enter Treatment Period 2.

- Eligible children who had the placebo injection procedure during Period 1 will now receive OAV101.
- Eligible children who received OAV101 will now have the placebo injection procedure.



What kind of tests will my child have?



Blood test



Breathing test



Heart activity



Heart imaging



Motor function tests



Neurological examination



Physical examination



Spinal tap



Urine test



X-ray

Your child will not have all of the tests at each visit. There may also be some additional tests. Talk to the study doctor for more information.