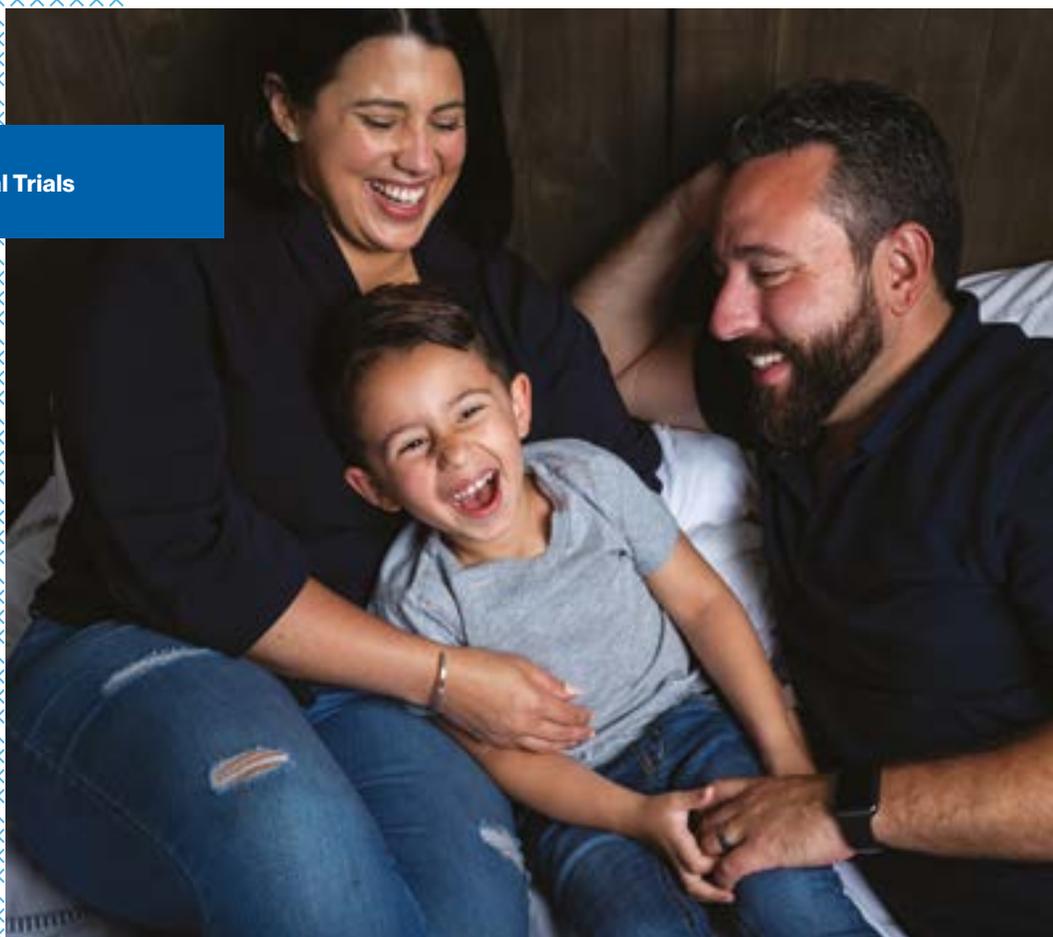


Clinical Trials



The STEER OAV101B12301 Schedule and Planner

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

Thank you for joining the STEER Study.

This study is testing an investigational gene therapy called OAV101 to see if it is safe and can improve symptoms and movement in children age 2 to less than 18 years with Type 2 spinal muscular atrophy (SMA).

This booklet explains what will happen at study visits. It also has space for you to keep track of scheduled appointments and helpful information. It is important that your child attends all study visits so the doctor can keep an eye on his/her health and understand how OAV101 may be affecting your child's body.

Your participation is greatly appreciated. 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.

As with all medicines, there is a chance for side effects. Your child will be closely monitored during the entire study.

The study team will happily answer any questions you have. Remember, participation is voluntary and you and your child can choose to leave the study at any time.

With much appreciation,

The STEER Study team

Study duration and visits

Your child will be in the study for up to 72 weeks (about 17 months) and have about 29 visits for health checks and tests. Most study visits may take up to 6 hours depending on the number and type of tests.

Your child will also have 2 hospital stays when receiving 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

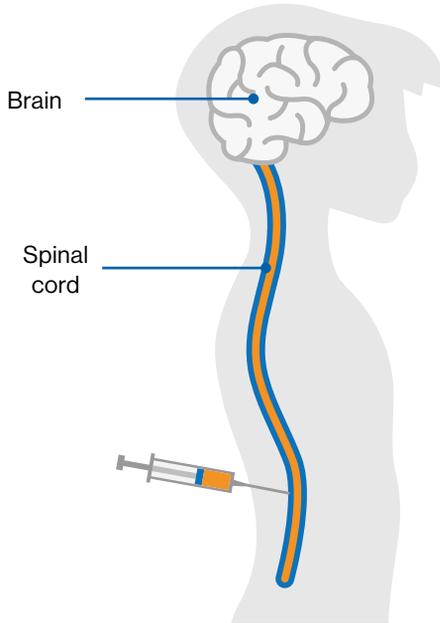
Follow-up Period 2

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.

Study treatment

OAV101 is given as a one-time injection into the lower part of the spine. This is called an **intrathecal injection**.



Study treatment groups

Your child will be assigned by chance to receive either **OAV101** gene therapy or a “**placebo injection procedure**.” You will not know which study treatment your child is receiving.

The placebo injection procedure is a needlestick to the lower back. OAV101 is not injected. It is important to have a placebo injection procedure to compare to OAV101 so the doctors can understand OAV101’s effects.

Both the OAV101 injection and the placebo injection procedure are performed in the hospital where your child will stay overnight for a few days.

Screening visits

Visit 1 Date: _____ Time: _____

Visit 2 Date: _____ Time: _____

Before starting the study, you will need to give permission for your child to join by reading and signing an **Informed Consent Form**. Depending on his/her age, your child may also give permission by signing an **Informed Assent Form**.



The study doctor will collect information and run tests to make sure the study is right for your child.

Health checks and tests



Blood test



Body mass



Breathing test



Head measurement



Heart activity



Heart imaging



Height



Medical history



Medicine review



Mental health check*



Movement ability



Neurological examination



Physical examination



Swallowing test



Urine test



Vital signs



Weight



X-ray

*Mental Health check may not be applicable (for <7yrs)

Baseline visit (Study start)

Visit 1 Date: _____ Time: _____

Your child will receive a steroid (prednisolone) or a placebo 24 hours before receiving the study treatment, depending on which group he/she is in.

Health checks and tests



Blood test



Body mass



Breathing test



Head measurement



Heart activity



Height



Medicine review



Movement ability



Movement monitoring*



Nasal swab



Neurological examination



Physical examination



Saliva test



Stool test



Swallowing test



Urine test



Vital signs



Weight

*Some participants may be offered a device like a wristwatch that tracks movement. This will be worn at home.

Treatment period (Hospital stay)

Date: _____ Time: _____

Your child will receive OAV101 or the placebo injection procedure in the hospital and stay overnight for a few days.

Health checks and tests



Blood test



Medicine review



Nasal swab



Neurological examination



Physical examination



Saliva test



Spinal tap



Stool test



Urine test



Vital signs



Weight

Your child will be continuously monitored throughout the study for any side effects from the study medicine.

Follow-up period 1 (1 year)

During this time, your child will have 18 visits for tests: once a week for the first month, once every 2 weeks from weeks 5 through 12, and then once every 4 weeks.

Your child will not have every test at each visit.

Health checks and tests



Blood test



Body mass



Breathing test



Heart activity



Heart imaging



Height



Improvement score



Medicine review



Mental health check**



Movement ability



Movement monitoring*



Nasal swab



Neurological examination



Physical examination



Questionnaire



Saliva test



Stool test



Swallowing test



Symptom score



Urine test



Vital signs



Weight



X-ray

Your child will be continuously monitored throughout the study for any side effects from the study medicine.

*Some participants may be offered a device like a wristwatch that tracks movement. This will be worn at home. **Mental health check may not apply to children younger than 7 years.

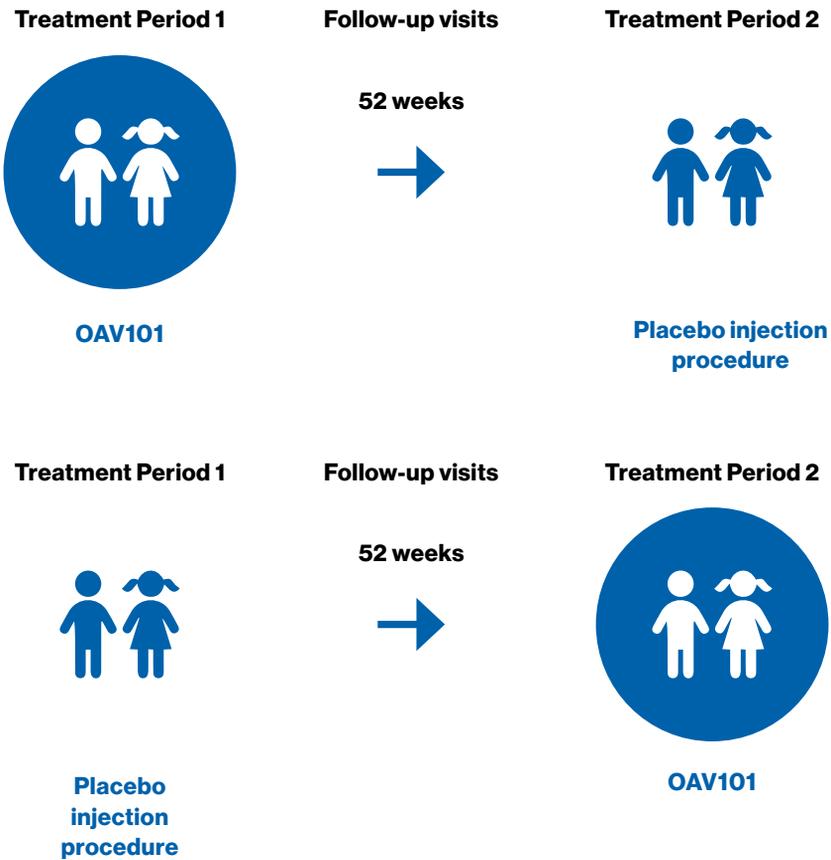
Follow-up period 1 (18 visits)

Use the chart below to keep track of your child’s scheduled visits and important information to share with the study doctor.

Visit	Week	Scheduled date/time	Notes and reminders
1	1		
2	2		
3	3		
4	4		
5	6		
6	8		
7	10		
8	12		
9	16		
10	20		
11	24		
12	28		
13	32		
14	36		
15	40		
16	44		
17	48		
18	52		

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 received OAV101 during Treatment Period 1 will have the placebo injection procedure in Treatment Period 2. Eligible children who had the placebo injection procedure during Treatment Period 1 will now receive OAV101.



Treatment period 2 (Hospital stay)

Date: _____ Time: _____

Your child will receive OAV101 or the placebo injection procedure in the hospital and will stay overnight for a few days.

Health checks and tests



Blood test



Medicine review



Nasal swab



Neurological examination



Physical examination



Saliva test



Spinal tap



Stool test



Urine test



Vital signs



Weight

Your child will be continuously monitored throughout the study for any side effects from the study medicine.

Follow-up period 2 (12 weeks)

During this time, your child will have 8 visits for tests: once a week, then once every 2 weeks. Your child will not have every test at each visit.

Health checks and tests



Blood test



Body mass



Heart activity



Heart imaging



Height



Improvement score



Medicine review



Mental health check**



Movement ability



Movement monitoring*



Nasal swab



Neurological examination



Physical examination



Questionnaire



Saliva test



Stool test



Swallowing test



Symptom score



Urine test



Vital signs



Weight

*Some participants may be offered a device like a wristwatch that tracks movement. This will be worn at home.

**Mental health check may not apply to children younger than 7 years.

Follow-up period 2 (8 visits)

Use the chart below to keep track of your child's scheduled visits and important information to share with the study doctor.

Visit	Week	Scheduled date/time	Notes and reminders
1	53		
2	54		
3	55		
4	56		
5	58		
6	60		
7	62		
8	64		

Study contact information

Study doctor's name:

Phone number:

Study coordinator's name:

Phone number:

Email:

Contact the study team if you have any questions or if you need to reschedule an appointment.

Glossary



Blood test: Samples of blood will be taken to look at blood cells and what the study medicine may be doing inside the body. Blood tests can measure how much study medicine is in the body and how it may be affecting other parts of the body. Participants who have started their periods will have their blood tested for pregnancy.



Body mass: The doctor will calculate how much body fat your child has based on his/her height and weight.



Breathing test: Your child will breathe into a tube attached to a computer to measure how much air he/she can breathe in and out of the lungs and how fast he/she can blow air out.



Head measurement: The study team will wrap a flexible measuring tape around your child's head to see how big it is.



Heart activity: A simple test called an electrocardiogram (ECG) will be used to check the electrical activity (rhythm) of your child's heart. For this test, your child will lie down and small, sticky pads (electrodes) will be attached to his/her chest. The pads are connected with wires to a computer.



Heart imaging: A painless test called an echocardiogram uses sound waves to see your child's heart pumping and beating inside the body.



Height: The study doctor will use a measuring tape to measure the length of your child's lower leg (from the knee to the ankle) to calculate how tall your child is.



Improvement score: The study team will use a scoring system to rate if and how your child's spinal muscular atrophy (SMA) is improving.



Informed consent: If you agree to have your child join the study, you will sign forms to give your permission. Your child may also give permission to join by signing an informed assent.



Medical history: The doctor will ask questions about your child's health, including his/her spinal muscular atrophy (SMA) and any surgeries he/she may have had.

Glossary



Medicine review: The study doctor will ask questions about medicines your child is taking now or has taken in the past.



Mental health check: Your child (if older than 7 years of age) will answer some questions about his/her mood and behavior and if he/she is feeling sad.



Movement ability: The study team will assess how well your child can perform certain movements. They will give your child scores based on his/her physical abilities.



Movement monitoring: Some participants may be offered a movement monitor to wear around the wrist while at home to keep track of their movements.



Nasal swab: The study doctor will collect a sample by gently rubbing the inside of your child's nose with a cotton swab.



Neurological examination: The study doctor will check to see how well different parts of your child's body are able to feel.



Physical examination: The doctor will check different parts of your child's body, such as the heart, lungs, muscles, and stomach.



Questionnaire: As your child's carer, you will fill out a questionnaire about how your child's spinal muscular atrophy (SMA) affects your everyday life.



Saliva test: Your child will spit into a cup and the sample will be tested to see what the treatment may be doing inside your child's body.



Spinal tap: A small amount of the fluid around your child's spine will be removed using a thin needle. The spinal fluid will be tested to see how your child's body may be responding to treatment.



Stool test: A small sample of your child's stool (poo) will be collected and tested to see what the treatment may be doing inside the body.



Swallowing test: A specialist will assess how well your child is able to eat and swallow.



Symptom score: The study team will use a scoring system to rate your child's symptoms of spinal muscular atrophy (SMA) and how they affect his/her ability to do everyday tasks.



Urine test: A sample of your child's urine will be tested for certain things such as sugar, protein, and blood. Participants who have started their period will also have their urine tested for pregnancy.



Vital signs: The study doctor will check your child's temperature, breathing rate, heart rate (pulse), and blood pressure to see how your child's heart is pumping.



Weight: The study doctor will use a scale to weigh your child without his/her shoes while he/she is wearing light clothing. Someone will help support your child on the scale while he/she is being weighed.



X-ray: An x-ray is used to take black-and-white pictures of the inside of the body. Your child will have an x-ray of his/her chest and back.

