Novartis - AVXS-101 Long-Term Follow-up

A Long Term Follow up Safety Study of Patients in the AVXS-101-CL-101 Gene Replacement Therapy Clinical Trial for Spinal Muscular Atrophy Type 1 Delivering AVXS 101

Summary

This is a long-term follow-up study for participants who have received the AVXS-101 (gene replacement therapy) infusion in a previous clinical trial. The study will investigate the long-term safety and effects of AVXS-101 in these participants. The participants will have annual face-to-face follow-up meetings for 5 years, and then annual phone-call meetings for 10 years.

Study Number: NCT03421977

Description by Novartis Gene Therapies

This is a long term, safety follow up study of patients in the AVXS-101-CL-101 gene replacement therapy clinical trial for SMA Type 1 delivering onasemnogene abeparvovec-xioi. Patients will roll over from the parent study into this long-term study for continuous safety monitoring for up to 15 years. The last visit of the parent study or early discontinuation from the parent study may serve as the visit at which the informed consent form process is conducted for the AVXS 101-LT-001 long term follow-up safety study. Patients will return annually for follow up study visits for five (5) years, and then will be contacted via phone annually for ten (10) years. Additionally, patient record transfers from their local physician and/or neurologist will be requested in conjunction with the annual study visits and phone contacts for review by the investigator.

If the patient is unable to return to the original investigative site, the sponsor will arrange with the patients' local established physician to serve as an additional investigator to conduct the required assessments.

Secondary Outcome Measures

Long-Term Safety [Time Frame: 15 years]

The primary objective is to collect long term safety data of patients with SMA Type 1 who were treated with onasemnogene abeparvovec-xioi in the AVXS-101-CL-101 gene replacement therapy clinical trial by assessing incidence of SAEs and Adverse Events of Special Interest.

Can I take part?

Inclusion Criteria

- Patient who received onasemnogene abeparvovec-xioi in the AVXS-101-CL-101 gene replacement therapy clinical trial for SMA Type 1.
- Parent/legal guardian willing and able to complete the informed consent process, comply with study procedures and visit schedule.

Exclusion Criteria

- Parent/legal guardian unable or unwilling to participate in the long term follow up safety study.

Other inclusion/exclusion criteria may apply.

For contact details and to find out more, please refer to ausnmd.org.

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AUSNMD

Trial Status
Fully recruited

Locations
Sydney - Children's Hospital, Fully recruited

Trial Sponsor
Novartis Gene Therapies

Age
Any age

SMASubtype
Type 1

SMN2 Copy Numbers Required
2 or more

Mode of delivery
IT

MRI
No

Phase
Observational

Length Of Participation
15 years

Recruitment Target
15

Therapeutic Category
Gene therapy