

# Long-term Follow-up of Patients With Spinal Muscular Atrophy Treated With OAV101 in Clinical Trials

Last Update: Dec 10, 2024

Long-term Follow-up of Patients With Spinal Muscular Atrophy Treated With OAV101 IT or OAV101 IV in Clinical Trials

ClinicalTrials.gov Identifier:

[NCT05335876](#)

Novartis Reference Number:COAV101A12308

[See if you Pre-qualify](#)

All compounds are either investigational or being studied for (a) new use(s). Efficacy and safety have not been established. There is no guarantee that they will become commercially available for the use(s) under investigation.

## Study Description

This is a global, prospective, multi-center study that is designed to assess the long-term safety and efficacy of OAV101 in patients who participated in an OAV101 clinical trial. The assessments of safety and efficacy in Study COAV101A12308 will continue for 5 years after enrollment in this study. The study is comprised of a Baseline Visit and 2 Follow-up Periods. For Follow-up Periods 1 and 2, which includes Baseline through Year 5 visits, assessments will be performed at the Investigational site. For the first 2 years (Follow-up Period 1), visits will occur every 6 months. For Years 3 to 5 (Follow-up Period 2) follow-up visits will be conducted annually. All patients will enter the study at the baseline visit and continue for 5 years.

Condition

Spinal Muscular Atrophy (SMA)

Phase

Phase3

Overall Status

Recruiting

Number of Participants

175

Start Date

Dec 19, 2022

Completion Date

Jun 26, 2030

Gender

All

Age(s)

0 Years - 100 Years (Child, Adult, Older Adult)

## Interventions

## **onasemnogene abeparvovec**

Onasemnogene abeparvovec is a non-replicating recombinant adeno-associated virus serotype 9 containing the human survival motor neuron gene under the control of the ytomegalovirus enhancer/chicken  $\beta$ -actin-hybrid promoter. Onasemnogene abeparvovec is administered as a one-time intravenous (IV) infusion or intrathecal (IT) injection. Dosage determined by participant weight.

## **Eligibility Criteria**

Inclusion Criteria:

1. Participated in an OAV101 clinical trial.
2. Written informed consent must be obtained before any assessment is performed.
3. Patient/Parent/legal guardian willing and able to comply with study procedures.

Exclusion Criteria:

There are no exclusion criteria for this study.

### **Australia**

#### **Novartis Investigative Site**

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### **Belgium**

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**Vietnam**

## Novartis Investigative Site

Recruiting

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## Worldwide Contacts

If the location of your choosing does not feature any contact detail, please reach out using the information below.

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