A Long-term Safety Study in Brazilian Patients With a Diagnosis of Spinal Muscular Atrophy Treated With Zolgensma

Last Update: Jan 15, 2025
A Long-term Safety Study in Brazilian Patients With a Confirmed Diagnosis of Spinal Muscular Atrophy (SMA) Treated With Onasemnogene Abeparvovec (Zolgensma®) - ARISER Study
ClinicalTrials.gov Identifier:
NCT06019637
Novartis Reference Number:COAV101ABR01
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

A long-term safety study in Brazilian patients with a confirmed diagnosis of Spinal Muscular Atrophy (SMA) treated with Onasemnogene Abeparvovec (Zolgensma®) This study is a non-interventional Post Authorization Safety Study (PASS) to evaluate long-term, real-world safety data of Brazilian pediatric patients diagnosed with SMA and treated with Onasemnogene Abeparvovec (Zolgensma®) for up to 15 years after the treatment. This study will support the benefit-risk assessment of Onasemnogene Abeparvovec in the approved indications and may also allow for detection of new safety signals and provide further guidance on the management of safety risks associated with Onasemnogene Abeparvovec to patients/caregivers, health care providers (HCPs) and treating physicians, as required by Brazilian Health Authority ANVISA as a conditional measure for granting Zolgensma®'s authorization.

Condition
Spinal Muscular Atrophies
Overall Status
Recruiting
Number of Participants
50
Start Date
Nov 22, 2023
Gender
All
Age(s)
0 Years - 100 Years (Child, Adult, Older Adult)

Interventions

Other

Onasemnogene Abeparvovec

Retrospective and prospective observational study. There is no treatment allocation.

Eligibility Criteria

Inclusion Criteria:

* Subject's parent or legal guardian has provided signed eICF.
* Subject with SMA, genetically confirmed: with a bi-allelic mutation in the SMN1 gene, and a clinical diagnosis of SMA Type 1 or up to 3 copies of the SMN2 gene.
* Subject treated* with Onasemnogene Abeparvovec (Zolgensma®) prior to enrolling in this study.

Subjects treated with nusinersen or risdiplam prior to Onasemnogene Abeparvovec (Zolgensma®) can be enrolled if currently not receiving it.

*Subjects can be enrolled in this study on the day treated with Onasemnogene Abeparvovec (Zolgensma®) or if prior medical history is available to complete all assessments retrospectively, in accordance with local ethical requirements.

* Subject and parent/guardian are willing and able to comply with the phone contacts through the course of the study

Exclusion Criteria:

* Patients currently enrolled in any interventional clinical trial** other than the phase IV OFELIA trial will be excluded from the study.

* Subjects who were enrolled in a clinical trial (independently of the disease indication and interventional treatment) but are not currently enrolled, can be included in this study.

During the follow-up, subjects who enroll any clinical trial with pharmacological intervention will discontinue from this study.

Brazil

Novartis Investigative Site

Recruiting

Curitiba,PR,81520-060,Brazil

Novartis Investigative Site

Recruiting

Sao Paulo,SP,05403-000,Brazil

Worldwide Contacts

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

Novartis Pharmaceuticals

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Source URL: *https://prod1.novartis.com/clinicaltrials/study/nct06019637*

List of links present in page

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