

Registry of Patients With a Diagnosis of Spinal Muscular Atrophy (SMA)

Status: RECRUITING

Eligibility Criteria

Sex: ALL

Age:

Healthy Volunteers: This study is NOT accepting healthy volunteers

Inclusion Criteria:

- Patients treated with OAV-101 with a genetically confirmed diagnosis of SMA regardless of the date of diagnosis.
- Appropriate consent/assent has been obtained for participation in the registry

Exclusion Criteria:

- Currently enrolled in an interventional clinical trial involving an investigational medicinal product to treat SMA.

Note: Patients who are participating in a Compassionate Use Program (CUP) for OAV-101 (Zolgensma) such as a Managed Access Program (MAP), an Expanded Access Program (EAP), Single Patient Investigational New Drug (IND) (SPI) or Named Patient Program (NPP) are eligible to enroll in the registry regardless of the date of a genetic or clinical diagnosis of SMA.

Conditions & Interventions

Interventions:

OTHER: Prospective observational registry, DRUG: Zolgensma

Conditions:

Spinal Muscular Atrophy (SMA)

More Information

Description:

Spinal muscular atrophy (SMA) is a neurogenetic disorder caused by a loss or mutation in the survival motor neuron 1 gene (SMN1) on chromosome 5q13, which leads to reduced SMN protein levels and a selective dysfunction of motor neurons. SMA is an autosomal recessive, early childhood disease with an incidence of 1:10,000 live births. SMA is the leading cause of infant mortality due to genetic diseases. The purpose of this registry is to assess the long term outcomes of patients with SMA in the context of advances in treatment options and also to characterize and assess long-term safety and effectiveness of OAV-101.

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Principal Investigator ID:

Phase:

IRB Number:

System ID: NCT04174157

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