

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

NCT04174157

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**Status** RECRUITING  
**Sponsor** Novartis Pharmaceuticals  
**Enrollment** 700 participants

## Key Eligibility Criteria

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### Inclusion (2)

- 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 (2)

- \- 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.

## Locations (99 total)

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Phoenix Children's Hospital, Phoenix, Arizona, United States  
Arkansas Children's Hospital, Little Rock, Arkansas, United States  
Loma Linda University Health, Loma Linda, California, United States  
... and 96 more locations

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<https://clinicaltrials.gov/study/NCT04174157>

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