

A Study to Assess the Safety, Tolerability, Efficacy, Pharmacokinetics, and Immunogenicity of Intravenous Administration of ARGX-119 in Pediatric Participants Aged 5 to Less Than 18 Years With Spinal Muscular Atrophy

NCT07287982

Status	RECRUITING
Phase	Phase 2
Sponsor	argenx
Enrollment	60 participants

Key Eligibility Criteria

Inclusion (5)

- Is aged 5 to <18 years when completing the informed consent process, defined as providing informed assent according to local regulations and having a parent or guardian sign the ICF, and can comply with protocol requirements.
- Has documented historical genetic diagnosis of 5q-SMA.
- Currently receiving a stable SMA treatment regimen (nusinersen or risdiplam) and/or have a history of onasemnogene abeparvovec treatment
- Must be able to walk at least 50 meters without walking aids in the 6MWT at screening

Exclusion (5)

- Known medical condition that would interfere with an accurate assessment of SMA, confound the results of the study, or put the participant at undue risk, as assessed by the investigator
- Recent major surgery, except spinal fusion, within 3 months of screening or intends to have major surgery during the study
- Current or previous administration of antimyostatin therapies in the past 6 months
- Severe scoliosis (defined as curvature $\gt 40^\circ$) and/or contractures at screening. o History of spinal fusion within 6 months before screening or planned during the study
- Respiratory insufficiency, defined by the medical necessity for invasive or noninvasive ventilation for daytime treatment while awake. Ventilation used overnight or during daytime naps is acceptable.

Locations (17 total)

Arkansas Children's Hospital, Little Rock, Arkansas, United States
Rady Childrens Hospital, San Diego, California, United States
Stanford University Medical Center, Stanford, California, United States
... and 14 more locations

<https://clinicaltrials.gov/study/NCT07287982>

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