ForPatients
by Roche

Spinal Muscular Atrophy (SMA)

A Study Evaluating the Effectiveness and Safety of Risdiplam Administered as an Early Intervention in Pediatric Participants With Spinal Muscular Atrophy After Gene Therapy

Trial Status	Trial Runs In	Trial Identifier
Recruiting	4 Countries	NCT05861986 2023-504508-26-00 BN44620

The information is taken directly from public registry websites such as ClinicalTrials.gov, EuClinicalTrials.eu, ISRCTN.com, etc., and has not been edited.

Official Title:

A Phase IV Open-Label Study Evaluating the Effectiveness and Safety of Risdiplam Administered as an Early Intervention in Pediatric Patients With Spinal Muscular Atrophy After Gene Therapy

Trial Summary:

This is an open-label, single-arm, multicenter clinical study to evaluate the effectiveness and safety of risdiplam administered as an early intervention in pediatric participants with spinal muscular atrophy (SMA) and 2 SMN2 copies who have previously received onasemnogene abeparvovec. Participants are children < 2 years of age genetically diagnosed with SMA.

Hoffmann-La Roche
Sponsor

Phase 4
Phase

NCT05861986 2023-504508-26-00 BN44620
Trial Identifiers

Eligibility Criteria:

Gender	Age	Healthy Volunteers
All	#3 Months & # 24 Months	No

Inclusion Criteria:

- <2 years of age at the time of informed consent

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- Confirmed diagnosis of 5q-autosomal recessive SMA, including genetic confirmation of homozygous deletion or compound heterozygosity predictive of loss of function of the Survival of Motor Neuron 1 (SMN1) gene
- Confirmed presence of two SMN2 gene copies as documented through laboratory testing
- Administration of onasemnogene abeparvovec pre-symptomatically or post-symptomatically
- Has received onasemnogene abeparvovec for SMA no less than 13 weeks, but not more than months 30 weeks, prior to enrollment
- If treated with risdiplam prior to onasemnogene abeparvovec, risdiplam treatment must not have exceeded 3 weeks and must be discontinued 1 day prior to onasemnogene abeparvovec administration
- Has, in the opinion of the investigator, not experienced clinically significant decline in function from the time of onasemnogene abeparvovec administration

Exclusion Criteria:

- Previous or current enrolment in investigational study prior to initiation of study treatment
- Any unresolved standard-of-care laboratory abnormalities per the onasemnogene abeparvovec prescribing information
- Concomitant or previous administration of an SMN2-targeting antisense oligonucleotide
- Concomitant or previous use of an anti-myostatin agent
- Participants requiring invasive ventilation or tracheostomy
- Participants requiring awake non-invasive ventilation or with awake hypoxemia (Arterial Oxygen Saturation [SaO2] <95%) with or without ventilator support
- Presence of feeding tube and an OrSAT score of 0
- Hospitalization for pulmonary event within the last 2 months, or any planned hospitalization at the time of screening
- Any major illness requiring hospitalization within 1 month before the screening examination or any febrile illness within 1 week prior to screening and up to first dose administration.

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