ForPatients
by Roche

Spinal Muscular Atrophy (SMA)

A Study Evaluating the Effectiveness and Safety of Risdiplam Administered in Pediatric Patients With Spinal Muscular Atrophy Who Experienced a Plateau or Decline in Function After Gene Therapy

Trial Status	Trial Runs In	Trial Identifier
Recruiting	5 Countries	NCT05861999 2023-505161-81-00 BN44621

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 in Pediatric Patients With Spinal Muscular Atrophy Who Experienced a Plateau or Decline in Function After Gene Therapy

Trial Summary:

This is an open-label, single-arm, multicenter clinical study to evaluate the effectiveness and safety of risdiplam administered in pediatric participants with SMA and 2 SMN2 copies who previously received onasemnogene abeparvovec and experience a plateau or decline in function. Participants to be enrolled are children <2 years of age genetically diagnosed with SMA.

Hoffmann-La Roche	Phase 4
Sponsor	Phase

NCT05861999 2023-505161-81-00 BN44621
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
- 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 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.
- In the opinion of the investigator, has demonstrated a plateau or decline in function post-gene therapy (with a duration of 26 weeks or less) documented by 2 individual time points in the functions as follows: swallowing AND one additional function/ability (respiratory, motor function, other) per appropriate expectation.

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
- 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.