

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
Recruiting

Trial Runs In
5 Countries

Trial Identifier
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
Sponsor

Phase 4
Phase

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Trial Identifiers

Eligibility Criteria:

Gender
All

Age
#3 Months & # 24 Months

Healthy Volunteers
No

Inclusion Criteria:

ForPatients

by Roche

- <2 years of age at the time of informed consent
- Confirmed diagnosis of 5q-autosomal recessive SMA
- Confirmed presence of two SMN2 gene copies
- Administration of onasemnogene abeparvovec pre-symptomatically or post-symptomatically
- Has received onasemnogene abeparvovec for SMA no less than 3 months prior to enrollment
- In the opinion of the investigator, has demonstrated a plateau or decline in function post-gene therapy (with a duration of 6 months 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:

- Treatment with investigational therapy prior to initiation of study treatment
- Any unresolved standard-of-care laboratory abnormalities per the onasemnogene abeparvovec prescribing information
- Concomitant or previous administration of a SMN2-targeting antisense oligonucleotide or SMN2 splicing modifier either in a clinical study or as part of medical care
- 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.