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
- 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, but not more than 7 months, prior to enrollment
- Has, in the opinion of the investigator, not experienced clinically significant decline in function from the time of onasemnogene abeparvovec administration

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
- Requiring awake non-invasive ventilation or with awake hypoxemia (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.