ForPatients
by Roche

Spinal Muscular Atrophy (SMA)

A Study to Investigate the Safety, Tolerability, Pharmacokinetics, Pharmacodynamics and Efficacy of RO7034067 in Type 2 and 3 Spinal Muscular Atrophy (SMA) Participants (SUNFISH)

Trial Status	Trial Runs In	Trial Identifier
Completed	16 Countries	NCT02908685 2016-000750-35 BP39055

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 Two Part Seamless, Multi-Center Randomized, Placebo-Controlled, Double-Blind Study to Investigate the Safety, Tolerability, Pharmacokinetics, Pharmacodynamics and Efficacy of Risdiplam (RO7034067) in Type 2 and 3 Spinal Muscular Atrophy Patients

Trial Summary:

Multi-center, randomized, double-blind, placebo-controlled study to assess the safety, tolerability, pharmacokinetics, pharmacodynamics, and efficacy of Risdiplam in adult and pediatric participants with Type 2 and Type 3 SMA. The study consists of two parts, an exploratory dose finding part (Part 1) of Risdiplam for 12 weeks and a confirmatory part (Part 2) of Risdiplam for 24 months.

Hoffmann-La Roche
Sponsor

Phase 2
Phase

NCT02908685 2016-000750-35 BP39055
Trial Identifiers

Eligibility Criteria:

Gender	Age	Healthy Volunteers
All	# 2 Years & # 25 Years	No

Inclusion Criteria:

- Confirmed diagnosis of 5q-autosomal recessive SMA

- Negative blood pregnancy test at screening and agreement to comply with measures to prevent pregnancy and restrictions on sperm donation
- For Part 1: Type 2 or 3 SMA ambulant or non-ambulant
- For Part 2: 1) Type 2 or 3 SMA non-ambulant; 2) RULM entry item A greater than or equal to 2; 3) ability to sit independently as assessed by item 9 of the MFM

Exclusion Criteria:

- Concomitant or previous participation in any investigational drug or device study within 90 days prior to screening, or 5 half-lives of the drug, whichever is longer
- Concomitant or previous administration of a SMN2-targeting antisense oligonucleotide, SMN2 splicing modifier or gene therapy either in a clinical study or as part of medical care
- Any history of cell therapy
- Hospitalization for a pulmonary event within the last 2 months or planned at time of screening
- Surgery for scoliosis or hip fixation in the one year preceding screening or planned within the next 18 months
- Unstable gastrointestinal, renal, hepatic, endocrine, or cardiovascular system diseases as considered to be clinically significant by the Investigator
- Presence of clinically significant electrocardiogram abnormalities before study drug administration from average of triplicate measurement or cardiovascular disease indicating a safety risk for participants as determined by the Investigator
- Any major illness within one month before the screening examination or any febrile illness within one week prior to screening and up to first dose administration
- Recently initiated treatment (within less than [<] 6 months prior to randomization) with oral salbutamol or another beta 2-adrenergic agonist taken orally
- Any prior use of chloroquine, hydroxychloroquine, retigabin, vigabatrin or thioridazine, is not allowed
- Ascertained or presumptive hypersensitivity (e.g., anaphylactic reaction) to Risdiplam or to the constituents of its formulation
- Recent history (less than one year) of ophthalmological diseases
- Participants requiring invasive ventilation or tracheostomy