ForPatients
by Roche

Spinal Muscular Atrophy (SMA)

Investigate Safety, Tolerability, PK, PD and Efficacy of RO7034067 in Infants With Type1 Spinal Muscular Atrophy (FIREFISH)

Trial Status	Trial Runs In	Trial Identifier
Completed	16 Countries	NCT02913482 2016-000778-40 BP39056

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, Open-label, Multicenter Study to Investigate the Safety, Tolerability, Pharmacokinetics, Pharmacodynamics and Efficacy of Risdiplam (RO7034067) in Infants With Type 1 Spinal Muscular Atrophy

Trial Summary:

Open-label, multi-center clinical study is to assess the safety, tolerability, pharmacokinetic (PK), pharmacodynamics (PD), and efficacy of Risdiplam (RO7034067) in infants with Type 1 spinal muscular atrophy (SMA). The study consists of two parts, an exploratory dose finding part (Part 1) and a confirmatory part (Part 2) which will investigate Risdiplam (RO7034067) for 24-months at the dose selected in Part 1.

Hoffmann-La Roche
Sponsor

Phase 2
Phase

NCT02913482 2016-000778-40 BP39056
Trial Identifiers

Eligibility Criteria:

Gender	Age	Healthy Volunteers
All	# 1 Month & # 7 Months	No

Inclusion Criteria:

- Clinical history, signs or symptoms attributable to Type 1 SMA with onset after 28 days but prior to the age of 3 months
- Gestational age of 37 to 42 weeks

- Confirmed diagnosis of 5q-autosomal recessive SMA
- Participants has two survival motor neuron 2 (SMN2) gene copies, as confirmed by central testing
- Body weight greater than or equal to (>=) third percentile for age, using appropriate country-specific guidelines
- Receiving adequate nutrition and hydration (with or without gastrostomy) at the time of screening, in the opinion of the Investigator
- Adequately recovered from any acute illness at the time of screening and considered well-enough to participate in the opinion of the Investigator

Exclusion Criteria:

- Concomitant or previous participation in any investigational drug or device study within 90 days prior to screening or 5 half-lives, whichever is longer
- Concomitant or previous administration of SMN2-targeting antisense oligonucleotide, SMN2 splicing modifier or gene therapy study
- Any history of cell therapy
- Hospitalization for pulmonary event within the last 2 months, or planned at the time of screening
- Presence of clinically relevant electrocardiogram (ECG) abnormalities before study drug administration
- Unstable gastrointestinal, renal, hepatic, endocrine or cardiovascular system diseases
- Participants requiring invasive ventilation or tracheostomy
- Participants requiring awake non-invasive ventilation or with awake hypoxemia (arterial oxygen saturation less than [<] 95 percent [%]) with or without ventilator support
- Participants with a history of respiratory failure or severe pneumonia, and have not fully recovered their pulmonary function at the time of screening
- Multiple or fixed contractures and/or hip subluxation or dislocation at birth
- Presence of non-SMA related concurrent syndromes or diseases
- 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
- Any inhibitor of cytochrome P450 (CYP) 3A4 and/or any Organic Cation Transporter 2 (OCT-2) and multidrug and toxin extrusion (MATE) substrates taken within 2 weeks and/or any inducer of CYP3A4 taken within 4 weeks (or within 5-times the elimination half-life, whichever is longer) prior to dosing or participants (and the mother, if breastfeeding the infant) taking any nutrients known to modulate CYP3A activity and any known flavin containing monooxygenase (FMO) 1 or FMO3 inhibitors or substrates
- Prior use (at any time in the participants lives) and/or anticipated need for quinolones (chloroquine and hydroxychloroquine), thioridazine, vigabatrin, retigabine, or any other drug known to cause retinal toxicity during the study. Infants exposed to chloroquine, hydroxycholoroquine, thioridazine, vigabatrin, retigabine or drugs with known retinal toxicity given to mothers during pregnancy (and lactation) should not be enrolled.
- Recent history (less than 6 months) of ophthalmic disease that would interfere with the conduct of the study as assessed by an ophthalmologist
- Therapeutic use, defined as use for 8 weeks or longer, of the following medications within 90 days prior to enrollment: riluzole, valproic acid, hydroxyurea, sodium phenylbutyrate, butyrate derivatives, creatine, carnitine, growth hormone, anabolic steroids, probenecid, agents anticipated to increase or decrease muscle strength, agents with known or presumed histone deacetylase (HDAC) inhibitory effect, medications known to or suspected of causing retinal toxicity (deferoxamine, topiramate, latanoprost, niacin, rosiglitazone, tamoxifen, canthaxanthine, sildenafil, and interferon) and medications with known phototoxicity liabilities (e.g., oral retinoids including over-the-counter [OTC] formulations, amiodarone, phenothiazines and use of minocycline)