

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
Completed

Trial Runs In
16 Countries

Trial Identifier
NCT02913482 2016-000778-40
BP39056

The source of the below information is the publicly available website [ClinicalTrials.gov](https://clinicaltrials.gov). It has been summarised and edited into simpler language.

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

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Trial Identifiers

Eligibility Criteria:

Gender
All

Age
>= 1 Month & <= 7 Months

Healthy Volunteers
No
