

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. 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

**NCT02913482 2016-000778-40 BP39056**  
Trial Identifiers

***Eligibility Criteria:***

**Gender**  
All

**Age**  
≥ 1 Month & ≤ 7 Months

**Healthy Volunteers**  
No