

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 Recruiting	Trial Runs In 1 Countries	Trial Identifier NCT05861986 2023-504508-26-00 BN44620
-----------------------------------	-------------------------------------	---------------------------------------------------------------------

The source of the below information is the publicly available website ClinicalTrials.gov. It has been summarised and edited into simpler language.

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 All	Age >=3 Months & <= 24 Months	Healthy Volunteers No
----------------------	-----------------------------------------	---------------------------------