ForPatients
by Roche

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	Trial Runs In	Trial Identifier
Recruiting	4 Countries	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	Phase 4
Sponsor	Phase

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Trial Identifiers

Eligibility Criteria:

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