

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

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Trial Identifiers

Eligibility Criteria:

Gender
All

Age
>=3 Months & <= 24 Months

Healthy Volunteers
No
