ForPatients
by Roche

Hemophilia A Severe Hemophilia A

A Study to Evaluate the Efficacy, Safety, Pharmacokinetics, and Pharmacodynamics of Subcutaneous Emicizumab in Participants From Birth to 12 Months of Age With Hemophilia A Without Inhibitors

Trial Status	Trial Runs In	Trial Identifier
Active, not recruiting	14 Countries	NCT04431726 2020-001733-12,2023-505964-13-00 MO41787

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 a Phase IIIb, multicenter, open-label, single-arm study of prophylactic emicizumab in previously untreated and minimally treated patients at study enrollment from birth to #12 months of age with severe hemophilia A (intrinsic factor VIII [FVIII] level <1%) without FVIII inhibitors. The study is designed to evaluate the efficacy, safety, pharmacokinetics, and pharmacodynamics of emicizumab administered at 3 milligrams per kilogram of body weight (mg/kg) once every 2 weeks (Q2W) for 52 weeks. After 1 year of treatment, participants will continue to receive emicizumab (1.5 mg/kg once every week [QW], 3 mg/kg Q2W or 6 mg/kg once every 4 weeks [Q4W]) over a 7-year long-term follow-up period under this study frame.

Hoffmann-La Roche	Phase 3
Sponsor	Phase

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Trial Identifiers

Eligibility Criteria:

Gender	Age	Healthy Volunteers
All	>=0 Months & <= 12 Months	No