ForPatients
by Roche

Hemophilia A

A Study to Evaluate the Efficacy, Safety, Pharmacokinetics, and Pharmacodynamics of Emicizumab Given Every 4 Weeks in Participants With Hemophilia A (HAVEN4)

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Trial Status	Trial Runs In	Trial Identifier
Completed	6 Countries	NCT03020160 2016-001094-33 HAVEN4 BO39182

The information is taken directly from public registry websites such as ClinicalTrials.gov, EuClinicalTrials.eu, ISRCTN.com, etc., and has not been edited.

Official Title:

A Multicenter, Open-Label, Phase III Study to Evaluate the Efficacy, Safety, Pharmacokinetics, and Pharmacodynamics of Emicizumab Given Every 4 Weeks (Q4W) in Patients With Hemophilia A

Trial Summary:

This multicenter, open-label, non-randomized study will assess the efficacy, safety, pharmacokinetics, and pharmacodynamics of emicizumab administered at a dose of 6 milligrams per kilogram (mg/kg) every 4 weeks in participants with hemophilia A with or without inhibitors against factor VIII (FVIII). The study consists of 2 parts: a pharmacokinetic (PK) run-in part followed by an expansion part.

Hoffmann-La Roche	Phase 3
Sponsor	Phase

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Trial Identifiers

Eligibility Criteria:

Gender	Age	Healthy Volunteers
All	#12 Years	No

Inclusion Criteria:

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- Body weight greater than or equal to (>/=) 40 kilograms (kg) at screening
- Diagnosis of severe congenital hemophilia A or hemophilia A with FVIII inhibitors
- Participants using rFVIIa or willing to switch to recombinant activated factor VII (rFVIIa) as primary bypassing agent for the treatment of breakthrough bleeds
- FVIII inhibitor test during screening with titer results available prior to first administration of study drug
- Participants without FVIII inhibitors, that is with less than (<) 0.6 Bethesda unit per milliliter [BU/mL];< 1.0 BU/mL only for laboratories with an historical sensitivity cutoff for inhibitor detection of 1.0 BU/mL, who completed successful immune tolerance induction (ITI) must have done so at least 5 years before screening and must have no evidence of inhibitor recurrence (permanent or temporary) indicated by detection of an inhibitor greater than (>) 0.6 BU/mL (> 1.0 BU/mL only for laboratories with an historical sensitivity cutoff for inhibitor detection of 1.0 BU/mL) since ITI
- Adequate hematologic, hepatic, and renal function

Exclusion Criteria:

- Inherited or acquired bleeding disorder other than hemophilia A
- Ongoing or planned ITI therapy; participants in whom ITI has failed will be eligible with a 72-hour washout period prior to the first emicizumab administration
- History of illicit drug or alcohol abuse within 48 weeks prior to screening, in the investigator's judgment
- Participants who are at high risk for thrombotic microangiopathy (TMA) (for example, have a previous medical or family history of TMA), in the investigator's judgment
- Previous (within the last 12 months) or current treatment for thromboembolic disease (with the exception of previous catheter-associated thrombosis for which anti-thrombotic treatment is not currently ongoing) or signs of thromboembolic disease
- Other conditions (for example, certain autoimmune diseases) that may currently increase the risk of bleeding or thrombosis
- History of clinically significant hypersensitivity associated with monoclonal antibody therapies or components of the emicizumab injection
- Known HIV infection with cluster of differentiation (CD) 4 cells counts <200 cells per microliter (cells/mcL)
- Use of systemic immunomodulators (for example, interferon) at enrollment or planned use during the study, with the exception of anti-retroviral therapy
- Concomitant disease, condition, significant abnormality on screening evaluations or laboratory tests, or treatment that could interfere with the conduct of the study, or that would, in the opinion of the investigator, pose an additional unacceptable risk in administering study drug to the participant
- Pregnancy or lactation or intention to become pregnant during the study
- Women with a positive serum pregnancy test result within 7 days prior to initiation of study drug

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