ForPatients
by Roche

Hemophilia A

A Study to Evaluate the Efficacy, Safety, and Pharmacokinetics of Prophylactic Emicizumab Versus no Prophylaxis in Hemophilia A Participants With Inhibitors (HAVEN1)

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Trial Status	Trial Runs In	Trial Identifier
Completed	14 Countries	NCT02622321 2015-002866-21 HAVEN1 BH29884

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 Randomized, Multicenter, Open-Label, Phase III Clinical Trial to Evaluate the Efficacy, Safety, and Pharmacokinetics of Prophylactic Emicizumab Versus no Prophylaxis in Hemophilia A Patients With Inhibitors

Trial Summary:

This multicenter, open-label study will evaluate the safety, efficacy and pharmacokinetics of prophylactic emicizumab treatment in participants previously treated with episodic or prophylactic bypassing agents. Episodic bypassing agent participants will be randomized in a 2:1 fashion to receive emicizumab prophylaxis (Arm A) versus no prophylaxis (Arm B) and will be stratified across Arms A and B according to the number of bleeds they experienced over the last 24 weeks prior to study entry (less than [<] 9 or greater than or equal to [>/=] 9 bleeds); Arm B participants will have the opportunity to switch to emicizumab prophylaxis after at least 24 weeks on-study. Prophylactic bypassing agent participants will switch to emicizumab prophylaxis (Arm C) from the start of the trial; enrollment will be extended for 24 weeks after the last participant has enrolled in Arms A or B or until approximately 50 participants have enrolled in Arm C, whichever occurs first. Episodic bypassing agent participants who previously participated in the non-interventional study BH29768 (NCT02476942) who were unable to enroll in Arms A or B, or participants on prophylactic bypassing agents who were unable to enroll in Arm C, prior to their closure will have the opportunity to enroll in Arm D. Like participants in Arms A and C, Arm D participants will receive emicizumab prophylaxis from the start of the trial. All participants will continue to receive episodic bypassing agent therapy to treat breakthrough bleeds, preferably with recombinant activated factor VII (rFVIIa).

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Hoffmann-La Roche
Sponsor

Phase 3
Phase

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Trial Identifiers

Eligibility Criteria:

Gender	Age	Healthy Volunteers
All	**#12 Years**	**No**

Inclusion Criteria:

- Body weight >/= 40 kilograms (kg) at the time of screening
- Diagnosis of congenital hemophilia A of any severity and documented history of high-titer inhibitor (that is [i.e.], >/= 5 Bethesda Units [BU])
- Documentation of treatment with episodic or prophylactic bypassing agents for at least the last 24 weeks
- >/= 6 bleeds in the last 24 weeks prior to screening (if on an episodic bypassing agent regimen) or >/=2 bleeds in the last 24 weeks prior to screening (if on a prophylactic bypassing agent regimen)
- Adequate hematologic, hepatic and renal function
- For women who are not postmenopausal or surgically sterile: agreement to remain abstinent or use single or combined highly effective contraceptive methods

Exclusion Criteria:

- Participants with inherited or acquired bleeding disorder other than hemophilia A
- Participants with ongoing (or plan to receive during the study) immune tolerance induction therapy or prophylaxis with Factor VIII (FVIII), with the exception of participants who have received a treatment regimen of FVIII prophylaxis with concurrent bypassing agent prophylaxis
- Previous (in the past 12 months) or current treatment for thromboembolic disease (with the exception of previous catheter-associated thrombosis for which antithrombotic treatment is not currently ongoing) or current signs of thromboembolic disease
- Participants with other conditions (for example [e.g.], certain autoimmune diseases) that may increase the risk of bleeding or thrombosis
- History of clinically significant hypersensitivity associated with monoclonal antibody therapies or components of the emicizumab injection
- Known human immunodeficiency virus (HIV) infection with cluster of differentiation 4 (CD4) count < 200 cells per microliter (cells/mcL) within 24 weeks prior to screening
- Use of systemic immunomodulators (e.g., interferon or rituximab) at enrolment or planned use during the study, with the exception of antiretroviral therapy
- Participants who are at high risk for thrombotic microangiopathy (TMA; e.g., have a previous medical or family history of TMA), in the investigator's judgment
- Concurrent disease, treatment, or abnormality in clinical laboratory tests that could interfere with the conduct of the study or that would, in the opinion of the investigator or Sponsor, preclude the participant's safe participation in and completion of the study or interpretation of the study results
- Planned surgery (excluding minor procedures such as tooth extraction or incision and drainage) during the study

- Receipt of emicizumab in a prior investigational study; An investigational drug to treat or reduce the risk of hemophilic bleeds within 5 half-lives of last drug administration; A non-hemophilia-related investigational drug within last 30 days or 5 half-lives, whichever is shorter; An investigational drug concurrently
- Unwillingness to use highly effective contraception methods for the specified duration in the protocol (females only, unless required otherwise by the local health authority)
- Clinically significant abnormality on screening evaluations or laboratory tests that, in the opinion of the investigator, may pose an additional risk in administering study drug to the participant
- Pregnancy or lactation, or intent to become pregnant during the study