ForPatients
by Roche

Hemophilia A

A Study to Evaluate the Safety and Tolerability of Prophylactic Emicizumab in Hemophilia A Patients With Inhibitors (STASEY)

A Study to Evaluate the Safety and Tolerability of Prophylactic Emicizumab in Hemophilia A Patients With Inhibitors

Trial Status	Trial Runs In	Trial Identifier
Completed	24 Countries	NCT03191799 2016-004366-25 STASEY MO39129

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 Single-Arm, Multicenter Phase IIIB Clinical Trial to Evaluate the Safety and Tolerability of Prophylactic Emicizumab in Hemophilia A Patients With Inhibitors

Trial Summary:

This is a phase IIIb, single arm, open-label, multi-center study to evaluate the safety and tolerability of emicizumab in participants with congenital hemophilia A who have documented inhibitors against Factor VIII (FVIII) at enrollment. Approximately 200 participants, aged 12 or older, will be enrolled in this study and are expected to be enrolled at approximately 85 sites globally. Participants will receive an initial weekly dose of prophylactic emicizumab subcutaneously for 4 weeks, followed by a weekly maintenance dose subcutaneously for the remainder of the 2-year treatment period.

Hoffmann-La Roche	Phase 3
Sponsor	Phase

NCT03191799 2016-004366-25 STASEY MO39129
Trial Identifiers

Eligibility Criteria:

Gender	Age	Healthy Volunteers
All	# 12 Years	No

Inclusion Criteria:

- As per investigator's judgement, a willingness and ability to comply with scheduled visits, treatment plans, laboratory tests, and other study procedures, including the patient-reported outcome (PRO) questionnaires and bleed diaries through the use of an electronic device or paper
- Aged 12 years or older at the time of informed consent
- Diagnosis of congenital hemophilia A with persistent inhibitors against FVIII
- Documented treatment with bypassing agents or FVIII concentrates in the last 6 months (on-demand or prophylaxis). Prophylaxis needs to be discontinued the latest by a day before starting emicizumab
- Adequate hematologic, hepatic, and renal function
- For women of childbearing potential: agreement to remain abstinent (refrain from heterosexual intercourse) or use a highly effective contraceptive method with a failure rate of <1% per year during the treatment period and for at least five elimination half-lives (24 weeks) after the last dose of emicizumab

Exclusion Criteria:

- Inherited or acquired bleeding disorder other than hemophilia A
- Ongoing (or plan to receive during the study) immune tolerance induction (ITI) therapy (prophylaxis regimens with FVIII and/or bypassing agents must be discontinued prior to enrollment). Patients receiving ITI therapy will be eligible following the completion of a 72-hour washout period prior to the first emicizumab administration
- History of illicit drug or alcohol abuse within 12 months prior to screening, as per the investigator's judgment
- High risk for thrombotic microangiopathy (TMA) (e.g., have a previous medical or family history of TMA), as per the investigator's judgment
- 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
- Other conditions (e.g., certain autoimmune diseases) that may increase the risk of bleeding or thrombosis
- History of clinically significant hypersensitivity reaction associated with monoclonal antibody therapies or components of the emicizumab injection
- Known human immunodeficiency virus (HIV) infection with CD4 count <200 cells/µL within 6 months prior to screening
- Use of systemic immunomodulators (e.g., interferon or rituximab) at enrollment or planned use during the study, with the exception of antiretroviral therapy
- 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 patient's safe participation in and completion of the study or interpretation of the study results
- Receipt of: Emicizumab in a prior investigational study; An investigational drug to treat or reduce the risk of hemophilic bleeds within five half-lives of last drug administration; A non-hemophilia-related investigational drug within last 30 days or five half-lives, whichever is shorter; or, Any concurrent investigational drug.
- Pregnancy or lactation, or intent to become pregnant during the study
- Positive serum pregnancy test result within 7 days prior to initiation of emicizumab (females only)