A Study to Assess the Efficacy and Safety of Emicizumab in Participants With Type 3 Von Willebrand Disease

Status: RECRUITING

Eligibility Criteria

Age: 1 month(s) and over

This study is NOT accepting healthy

Healthy Volunteers: volunteers

Inclusion Criteria:

* Confirmed diagnosis of Type 3 von Willebrand disease (VWD), based on medical records * Preexisting medical record verifying the status of von Willebrand factor (VWF) inhibitor (positive or negative, including titer if available) * Adequate hematologic, hepatic, and renal function * For participants of childbearing potential: agreement to remain abstinent or adhere to the contraception requirements Additional Inclusion Criteria for Arms A and B: * Age ≥1 month at the time of signing Informed Consent/Assent Form * Documented previous use of on-demand therapy with intermittent (less than once a week) on-demand SOC therapy for VWD * Having ≥2 treated bleeds (except menstrual bleeds) with factor concentrate within 24 weeks prior to enrollment Additional Inclusion Criteria for Arm C: * Age ≥2 years at the time of signing Informed Consent/Assent Form * Documented and confirmed previous use of SOC prophylactic therapy for VWD (1-3 times weekly, as per prescribed dose) as describec in the eligibility of Study WP45335 * Have completed all study requirements as defined in the WP45335 protocol for at least 24 weeks

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Exclusion Criteria:

* Inherited or acquired bleeding disorder other than Congenital Type 3 VWD * History of gastrointestinal bleeding within 18 months prior to enrollment, or any previous diagnosis of angiodysplasia * History of intracranial hemorrhage * Previous or current treatment for thromboembolic disease or signs of thromboembolic disease * Other conditions (e.g., certain autoimmune diseases) that may increase risk of bleeding or thrombosis * History of clinically significant hypersensitivity associated with monoclonal antibody therapies or components of the emicizumab injection * Use of systemic immunomodulators (e.g., interferon) at enrollment or planned use during the study, with the exception of anti-retroviral therapy

Conditions & Interventions

Interventions:
DRUG: Emicizumab, DRUG: von Willebrand Factor (VWF) Concentrates, DRUG: Factor VIII (FVIII) Concentrates, DRUG: von Willebrand Factor (VWF) and Factor VIII (FVIII) Concentrates, DRUG: Bypassing Agents

Conditions:
Von Willebrand Disease, Type 3

More Information

Contact(s): Reference Study ID Number: WP45338 https://forpatients.roche.com/ - global-roche-genentech-trials@gene.com

Principal Investigator:

IRB

Number:

System ID: NCT06998524
