

Hemophilia A

Clinical Trial of NXT007 for the Prophylactic Treatment of Hemophilia A in Infants and Children

Trial Status
Not yet recruiting

Trial Runs In

Trial Identifier
NCT07838194 2026-526103-29-00
BO45888

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, Single-Arm, Phase III Clinical Trial to Evaluate the Efficacy, Safety, Pharmacokinetics and Pharmacodynamics of NXT007 Prophylaxis in Pediatric Patients With Hemophilia A

Trial Summary:

The purpose of this study is to evaluate the efficacy, safety, pharmacokinetics and pharmacodynamics of NXT007 prophylaxis in pediatric patients aged 0 to 11 years with severe or moderate congenital hemophilia A without factor VIII inhibitors or congenital hemophilia A of any severity (severe, moderate, and mild) with inhibitors (emicizumab-naïve and treated). Patients with hemophilia A aged #12 to <18 years old with a body weight of <40 kilograms (kg) are also able to participate.

Hoffmann-La Roche
Sponsor

Phase 3
Phase

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Eligibility Criteria:

Gender
All

Age
#0 Years & # 17 Years

Healthy Volunteers
No

Inclusion Criteria:

- Age <12 years at the time of signing Informed Consent Form; or age #12 and <18 years with body weight <40 kg

ForPatients

by Roche

- Diagnosis of severe (Factor VIII coagulation protein activity [FVIII:C] <1 International Unit per decilitre [IU/dL]) or moderate (FVIII:C between #1 IU/dL and #5 IU/dL) congenital hemophilia A (HA) with or without inhibitors against factor VIII (FVIII)
- For potential participants with moderate HA without inhibitors, fulfillment of at least one of the following criteria: Current prophylaxis with long-term prophylaxis intention due to a severe bleed phenotype; For those not treated with prophylaxis: at least one traumatic joint or critical muscle bleed in the last 6 months or #2 spontaneous bleeds/year or 5 bleeds/year, including traumatic bleeds; Signs of joint degeneration compatible with hemophilic arthropathy (e.g., subchondral bone changes or the presence of synovitis) as assessed by any imaging assessment (e.g., ultrasound, MRI, radiograph); Bleeding at a critical site (e.g., intracranial).
- Diagnosis of mild (FVIII:C between >5 IU/dL and <40 IU/dL) congenital hemophilia A with chronic FVIII inhibitors, defined as documented FVIII inhibitor (#0.6 BU/mL or #1.0 BU/mL only for laboratories with a historical sensitivity cutoff for inhibitor detection of 1.0 BU/mL) and chronic reduction of endogenous baseline FVIII:C to <5 IU/dL for #12 months
- Documentation of the details of prophylactic and episodic FVIII treatment, bypassing agent (BPA) treatment, emicizumab prophylaxis treatment, and the number and type of bleeding episodes for at least the last 6 months prior to screening if appropriate
- For potential participants taking on-demand treatments prior to study entry: agreement to move to a prophylaxis treatment with NXT007
- Adequate renal, hepatic, and hematologic function, as defined in the protocol

Exclusion Criteria:

- Sensitivity to any of the study investigations, or components thereof, or drug or other allergy that, in the opinion of the investigator, contraindicates participation in the study
- Use of systemic immunomodulators (e.g., interferon or rituximab) at the time of enrollment or planned use during the study, except for antiretroviral therapy to treat HIV
- Refusal to accept plasma-derived and/or blood product transfusion support in an emergency scenario
- History or conditions, other than HA, which may indicate hypo- or hypercoagulopathy risk
- Planned surgery (excluding minor procedures, such as non-molar tooth extraction or incision and drainage) during the study
- History of ventricular dysrhythmias or risk factors for ventricular dysrhythmias such as structural heart disease (e.g., severe left ventricular systolic dysfunction, left ventricular hypertrophy)
- Any serious medical condition or abnormality in clinical laboratory tests that precludes an individual's safe participation in and completion of the study