ForPatients
by Roche

Hemophilia A

A clinical study to evaluate the effects of NXT007 compared to factor VIII prophylaxis in people with hemophilia A

A Clinical Study to Evaluate the Effects of NXT007 Compared to Factor VIII Prophylaxis in Participants With Hemophilia A

Trial Status	Trial Runs In	Trial Identifier
Recruiting	18 Countries	NCT07416526 2025-522434-32-00 2025 WO45886

The clinical trial information herein is sourced from public registries such as ClinicalTrials.gov, EuClinicalTrials.eu, and ISRCTN.com. Additional patient-friendly (lay) content has been prepared by Roche to support understanding.

Official Title:

A Multicenter, Randomized, Open-Label, Phase III Clinical Trial to Evaluate the Efficacy, Safety, Pharmacokinetics, and Pharmacodynamics of NXT007 Prophylaxis Versus Factor VIII Prophylaxis in People With Hemophilia A Without Inhibitors

Trial Summary:

The purpose of this study is to evaluate the efficacy, safety, pharmacokinetics and pharmacodynamics of NXT007 prophylaxis compared with Factor VIII (FVIII) prophylaxis in participants with severe or moderate congenital hemophilia A without FVIII inhibitors. The study will include people aged #12 years old who have been on FVIII prophylaxis treatment prior to study entry.

Hoffmann-La Roche	Phase 3
Sponsor	Phase

NCT07416526 2025-522434-32-00 2025 WO45886
Trial Identifiers

Eligibility Criteria:

Gender	Age	Healthy Volunteers
All	#12 Years	No

1. Why is this study needed?

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Haemophilia A (HA) is a health condition where the blood doesn't clot as it should. People with HA don't have enough of a specific blood clotting protein, called factor 8. This makes them bleed for a longer time after they get a cut or injury. It can cause spontaneous bleeding inside joints (e.g. knees, elbows, ankles) and muscles, resulting in pain and difficulty with physical activities. Bleeding in affected joints can lead to arthritis, starting as young as school age. Sometimes, bleeding in the brain or other parts of the body (such as the gut) can happen requiring immediate medical attention. Preventing bleeds and keeping joints healthy are key goals in the treatment of hemophilia.

Current treatments to reduce risk of bleeds (prophylaxis) include regular infusions into a vein of factor 8 medicine - these need to be given every few days and treatment stops working in some people who develop ‘inhibitors’ against it. A ‘non-factor’ medicine such as emicizumab can also be used, which copies the way factor 8 works and is given as injections under the skin every 1 to 4 weeks. However, whichever current treatment is used, bleeds can still sometimes happen, and extra treatment is needed if they are injured or need surgery. Better treatments are needed to help people with HA live more freely, without the risk, worry, and effects of bleeding.

This study is testing a medicine called NXT007. It is being developed to treat HA. NXT007 is an experimental medicine. This means health authorities (like the U.S. Food and Drug Administration and European Medicines Agency) have not approved NXT007 for the treatment of HA.

This study aims to compare how NXT007 provides protection from bleeding compared to factor 8.

2. Who can take part in the study?

People (males/females) of 12 years of age or above with HA can take part in the study if they have severe or moderate HA, do not have inhibitors against factor 8 and weigh 40 kg or more. They must also have been on factor 8 to prevent bleeds at least 6 months prior to starting the study.

People may not be able to take part in this study if they have previously received NXT007 at any time, or received other HA treatments, like emicizumab in the 6 months prior to starting the study. They also may not be able to join if they have planned complex surgery, certain other medical conditions including risk of bloodclotting (thrombosis), or are taking certain medicines that affect the immune system. People who are pregnant, trying to get pregnant, or currently breastfeeding cannot take part in the study.

3. How does this study work?

People will be screened to check if they are able to participate in the study. The screening period will take place from 28 days before the start of treatment.

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Everyone who joins this study will be split up into 2 groups randomly (like throwing a dice) and given either:

- NXT007 (Group 1), as an injection under the skin (subcutaneous injection) using a special penlike device that helps give medicine quickly and easily (autoinjector), with 2 administrations in the first month, followed by approximately monthly administrations for 6 months, or
- Factor 8 (Group 2), as an injection into the vein, following the schedule agreed by the participants and the doctor, for 6 months

This is an open-label study. This means everyone involved, including the participant and the study doctor, will know the study treatment the participant has been given.

During the main treatment period of the study, the study doctor will see participants 2 times in the first month, and then once a month after that. They will see how well the treatment is working and any unwanted effects participants may have. During these visits, the study doctor will check on the participant's well-being. After completing the main treatment period, all participants will have the option to take part in an extension of the study. Participants who received NXT007 during the main treatment period (Group 1) will continue receiving it once a month. Those who received factor 8 (Group 2) will be able to start NXT007 with 2 administrations in the first month, followed by monthly administration thereafter. The participants will continue to have clinic visits, so the study doctor will continue to check on the participant's well-being. The total time of participation in the study will be about 4 years. Participants have the right to stop study treatment and leave the study at any time, if they wish to do so. In that case, participants will have 3 follow-up visits 1, 6, and 12 months after the last NXT007 treatment, or 1 follow-up visit 28 days after the last factor 8 treatment period visit if the participant does not wish to start NXT007 in the treatment extension.

4. What are the main results measured in this study?

The main results measured in the study to assess if the medicine has worked are the number of bleeding episodes participants may have in a year that need treatment. Other key results measured in the study include

- The number of 'all' bleeds participants have (whether a treatment is given or not)
- The number of bleeds over time with no clear cause that need treatment
- The number of bleeds in the joints over time that need treatment
- How a person's health and HA symptoms impact their daily life and their ability to function and enjoy life.
- The number and seriousness of unwanted effects

5. Are there any risks or benefits in taking part in this study?

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Taking part in the study may or may not make participants feel better. But the information collected in the study can help other people with similar health conditions in the future.

It may not be fully known at the time of the study how safe and how well the study treatment works. The study involves some risks to the participant. But these risks are generally not greater than those related to routine medical care or the natural progression of the health condition. People interested in taking part will be informed about the risks and benefits, as well as any additional procedures or tests they may need to undergo. All details of the study will be described in an informed consent document. This includes information about possible effects and other options of treatment.

Risks associated with the study medicines
Participants may have unwanted effects of the medicines used in this study. These unwanted effects can be mild to severe, even life-threatening, and vary from person to person. During this study, participants will have regular check-ups to see if there are any unwanted effects.

NXT007 : NXT007 has had limited testing in humans. Therefore, the unwanted effects of this medicine are not fully known now. Participants will be told about the possible unwanted effects of NXT007 based on laboratory studies or knowledge of similar medicines. Known unwanted effects include loss of bleed protection due to antibodies against NXT007.

Known unwanted effects of injection include a reaction on the skin where it has been pricked with a needle to give a treatment.

Factor 8: Known unwanted effects include allergic reaction, and development of proteins called ‘inhibitors’ that reduce how well the treatment works.

The study medicines may be harmful to an unborn baby. Women must take precautions to avoid exposing an unborn baby to the study treatment.

Inclusion Criteria:

- Diagnosis of severe (FVIII:C <1 IU/dL [International Unit per decilitre]) or moderate (FVIII:C between #1 IU/dL and #5 IU/dL) congenital hemophilia A without inhibitors against FVIII
- No documented inhibitor (i.e., <0.6 BU/mL [Bethesda unit per millilitre]), FVIII half-life #6 hours, or FVIII recovery >66% in the last 3 years prior to screening
- Documented historical negative test for FVIII inhibitor (i.e., <0.6 BU/mL) within 12 months prior to enrollment
- Documentation of the details of prophylactic and episodic FVIII treatment and of the number and type of bleeding episodes for at least the last 6 months prior to screening
- Agreement to adhere to the contraception requirements (for potential participants with childbearing potential)

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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 anti-retroviral therapy to treat HIV
- Planned surgery (excluding minor procedures such as non-molar tooth extraction, incision and drainage) during the study
- History or presence of an abnormal ECG that is deemed clinically significant, (e.g., complete left bundle branch block, second- or third- degree atrioventricular heart block) or ECG evidence or clinical history of prior myocardial infarction
- Refusal to accept plasma-derived and/or blood product transfusion support in an emergency scenario
- 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), coronary heart disease (symptomatic or with ischemia demonstrated by diagnostic testing)

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