ForPatients
by Roche

Hämophilie A

A Clinical Study to Evaluate the Effects of NXT007 Compared to Emicizumab Prophylaxis in People With Hemophilia A

Trial Status	Trial Runs In	Trial Identifier
Rekrutierung läuft	19 Countries	NCT07416604 2025-522435-33-00 2025 BO45887

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Official Title:

A Multicenter, Randomized, Open-Label, Phase III Clinical Trial to Evaluate the Efficacy, Safety, Pharmacokinetics, and Pharmacodynamics of NXT007 Prophylaxis Versus Emicizumab Prophylaxis in People With Hemophilia A

Trial Summary:

The purpose of this study is to evaluate the efficacy, safety, pharmacokinetics, and pharmacodynamics of NXT007 prophylaxis compared with emicizumab prophylaxis in people age 12 years and older with severe or moderate congenital hemophilia A without factor VIII (FVIII) inhibitors or with hemophilia A of any severity (severe, moderate, and mild) with FVIII inhibitors.

Hoffmann-La Roche
Sponsor

Phase 3
Phase

NCT07416604 2025-522435-33-00 2025 BO45887
Trial Identifiers

Eligibility Criteria:

Gender	Age	Healthy Volunteers
All	#12 Years	No

1. Why is this study needed?

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 protects from bleeding compared with emicizumab in people with HA.

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 with or without inhibitors against factor 8. They can also join if they have mild HA and have had inhibitors against factor 8 for more than a year.

People who have previously been treated with emicizumab can also take part. They must also weigh 40 kg or more. People may not be able to take part in this study if they have recently received other experimental medicines for HA treatment . They also may not be able to join if they have planned complex surgery, certain medical conditions including the risk of blood clotting (thrombosis), or if they 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.

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

- NXT007 (Group 1), as an injection under the skin (subcutaneous injection), with 2 administrations in the first month, followed by approximately monthly administrations, or
- Emicizumab (Group 2), as an injection under the skin, every week during the first month of the study for participants who have not previously received emicizumab. Then the dosing schedule (every 1, 2, Page 2 of 2 Lay CTD v1.0 (02.09.2025) [EN-GB] based on the Lay CTD template v4.0 dated 15.01.2024 or 4 weeks) will be decided together with the participant and the study doctor and continued for at least 6 months. Participants already using emicizumab will continue their usual schedule for at least 7 months.

Participants will have an equal chance of being placed in either group.

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 this study, the study doctor will see participants up to 4 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 emicizumab (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.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 after 1, 6, and 12 months of completing the NXT007 treatment, and 1 follow-up visit at 6 months after the emicizumab treatment 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 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?

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.

Emicizumab: Known unwanted effects include pain or discomfort where the injection was administered, headache and joint pain. For both medicines, known unwanted effects of injection include a reaction on the skin where it has been pricked with a needle to give a treatment.

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 International Unit per decilitre [IU/dL]) or moderate (FVIII:C between #1 IU/dL and #5 IU/dL) congenital hemophilia A with or without inhibitors against FVIII
- 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
- Documented historical FVIII inhibitor assay results within the 12 months prior to enrollment

- 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
- For potential participants taking on-demand treatments prior to study entry: agreement to move to a prophylaxis treatment with either emicizumab or NXT007, according to assigned randomization

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
- 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), coronary heart disease (symptomatic or with ischemia demonstrated by diagnostic testing)
- 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 evidence or clinical history of prior myocardial infarction