ForPatients
by Roche

Hemophilia A

A clinical trial to look at how safely and how well NXT007 works at different doses in people with hemophilia A – and to understand how the body processes NXT007

A Study to Evaluate the Safety, Tolerability, Pharmacokinetics, Pharmacodynamics, and Efficacy of NXT007 in Persons With Severe or Moderate Hemophilia A

Trial Status	Trial Runs In	Trial Identifier
Recruiting	6 Countries	NCT05987449 2023-503906-35-00 WP44714

The source of the below information is the publicly available website ClinicalTrials.gov. It has been summarised and edited into simpler language.

Trial Summary:

WP44714 is a Phase I/II, open-label, non-randomized, global, multicenter trial consisting of two parts: * Part 1 is a multiple-ascending dose (MAD) study in adult and adolescent male participants with severe or moderate hemophilia A with or without factor VIII (FVIII) inhibitors. * Part 2 is a multiple-dose study in pediatric male participants with severe or moderate hemophilia A with or without FVIII inhibitors. The overall aim of the study is to investigate the safety, tolerability, pharmacokinetics, pharmacodynamics, immunogenicity, and efficacy of NXT007.

Hoffmann-La Roche	Phase 1/Phase 2
Sponsor	Phase

NCT05987449 2023-503906-35-00 WP44714
Trial Identifiers

Eligibility Criteria:

Gender	Age	Healthy Volunteers
Male	>=2 Years & <= 59 Years	No

1. Why is the WP44714 clinical trial needed?

Hemophilia A is a genetic disease caused by a missing or defective blood clotting protein called factor 8 (often written as Factor VIII, or FVIII) causing people to bleed for longer than people without hemophilia. It can cause spontaneous bleeding inside joints

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(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 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 in people with hemophilia, and extra treatment is needed if they are injured or need surgery. Better treatments are needed to help people with hemophilia A live more freely from the risk and worry of bleeding.

NXT007 is an experimental drug, which means health authorities have not approved it for treating hemophilia A, but researchers hope it will work better than current treatments with higher levels of protection against bleeds. This clinical trial aims to test the safety of NXT007, to understand how the body processes NXT007, and to measure the effects, good or bad, of NXT007 in people with hemophilia A.

2. How does the WP44714 clinical trial work?

This clinical trial is recruiting people with the severest forms of hemophilia (either moderate or severe hemophilia A). The study will have two Parts. Part 1 is for people 12–59 years old, Part 2 is for children 2 to 11 years old. Participants in either Part 1 or Part 2 of this clinical trial will be given NXT007 for at least 6 months, and the clinical trial team will see them regularly during this time These visits will include checks to see how the participant responds to the treatment and to review and record any side effects they may have. In Part 1 only, participants will need to stay at the hospital for at least 2 nights when being given the first dose. This is because NXT007 is a new drug and different doses will be tested. After 6 months of treatment, participants may choose to be given NXT007 for as long as it helps them for up to 7 years in an extension phase of the trial. If participants choose not to continue treatment in the extension phase, the total time in the clinical trial will be 6 months, with a follow-up time of 1 year. Participants can stop trial treatment and leave the clinical trial at any time – this will not impact their normal hemophilia care.

3. What are the main endpoints of the WP44714 clinical trial?

Clinical trial endpoints are the main results measured in a trial. To see how safe NXT007 is and if it works as expected, the main endpoints measured in this trial are the number, type and seriousness of any side effects. The other clinical trial endpoints include how the body processes NXT007, whether the participant's immune system tries to reject the NXT007, and the number of bleeds.

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4. Who can take part in this clinical trial?

People can take part in this trial if they are male, 12–59 years old and weigh at least 40 kg for Part 1 or 2–11 years old, weigh less than 40 kg for Part 2 and have moderate or severe hemophilia A. For Part 1, people will not be able to take part in this trial if they have previously received certain other treatments including emicizumab. For both Parts of the trial, people will not be able to take part in the trial if they (or their close family members in some cases) have certain other medical conditions including risk of blood-clotting (thrombosis). The clinical trial team will check that people meet the criteria before they join the trial.

5. What treatment will participants be given in this clinical trial?

In Part 1, participants who do not have an inhibitor will receive a 'pre-treatment' dose of their usual factor 8 medicine at a convenient time for them in the month before receiving NXT007. This is to record their body's response to their usual factor treatment. Then, all participants will be placed into groups and given different doses of NXT007 as an injection under the skin (subcutaneous injection). The first 3 doses of NXT007 will be given every 2 weeks, and all following doses will be given every 4 weeks. The first 2 doses of NXT007 will be higher than the remaining doses – this is to reach a high level of NXT007 in the body as soon as possible. The dose given will depend on when participants join the trial and the safety results from the previous dose group. The first group of participants will be given the lowest dose of NXT007, and later groups will be given increasingly higher doses of NXT007. Participants will stay on the same dose of NXT007 throughout the trial, including into the extension period, even though other later participants may be on a higher dose. In Part 2, people will be given NXT007 doses that have already been tested in people 12–59 years old (Part 1). This is an open-label trial, which means everyone involved, including the participant and the clinical trial team, will know the clinical trial treatment the participant has been given.

6. Are there any risks or benefits in taking part in this clinical trial?

The safety and effectiveness of a new medicine may not be fully known at the time of the trial. Most trials involve some risks to the participant. However, it may not be greater than the risks related to routine medical care or the natural progression of the health condition. People who would like to participate will be told about any possible risks and benefits of taking part in the clinical trial, as well as any additional procedures, tests, or assessments they will be asked to undergo. All of these will be described in an informed consent document (a document that provides people with the information they need to decide to volunteer for the clinical trial).

Risks associated with the clinical trial drug Participants may have side effects (an unwanted effect of a drug or medical treatment) from NXT007. Some participants may experience no side effects. Side effects can be mild to severe, even life-threatening, and

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vary from person to person. Participants will be closely monitored during the clinical trial and safety assessments will be performed regularly. NXT007 is being given to a small number of people in a parallel trial taking place in several Asian countries. Participants will be told about any known or possible side effects of NXT007 or subcutaneous injections, based on human and laboratory studies or knowledge of similar drugs.

Potential benefits associated with the clinical trial Participants' hemophilia may or may not improve by participating in the clinical trial. Still, the information collected may help other people with similar medical conditions in the future.

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