ForPatients
by Roche

Hemofilia A

Wieloo#rodkowe, prowadzone metod# otwartej próby badanie oceniaj#ce bezpiecze#stwo, skuteczno##, farmakokinetyk# i farmakodynamik# emicyzumabu u pacjentów z #agodn# lub umiarkowan# hemofili# a bez inhibitorów czynnika VIII

A Study to Evaluate the Safety, Efficacy, Pharmacokinetics and Pharmacodynamics of Emicizumab in Participants With Mild or Moderate Hemophilia A Without FVIII Inhibitors

Trial Status	Trial Runs In	Trial Identifier
Aktywne, nie rekrutuje	10 Countries	NCT04158648 2023-506610-52-00 BO41423

Informacje pochodz# bezpo#rednio ze stron internetowych rejestrów publicznych, takich jak ClinicalTrials.gov, EuClinicalTrials.eu, ISRCTN.com itp. i nie by#y edytowane.

Official Title:

WIELOO#RODKOWE, PROWADZONE METOD# OTWARTEJ PRÓBY BADANIE OCENIAJ#CE BEZPIECZE#STWO, SKUTECZNO##, FARMAKOKINETYK# I FARMAKODYNAMIK# EMICYZUMABU U PACJENTÓW Z #AGODN# LUB UMIARKOWAN# HEMOFILI# A BEZ INHIBITORÓW CZYNNIKA VIII

Trial Summary:

F. Hoffmann-La Roche Ltd
Sponsor

Badanie fazy III
Phase

NCT04158648 2023-506610-52-00 BO41423
Trial Identifiers

Eligibility Criteria:

Gender	Age	Healthy Volunteers
All		No

How does the HAVEN 6 clinical trial work?
This clinical trial is recruiting people who have hemophilia A. In order to take part, patients must have mild or moderate hemophilia A (without factor FVIII antibodies), and be suitable to receive treatment for bleed prevention (prophylaxis).

The purpose of this study is to test the effects, good and/or bad, of emicizumab in patients with mild or moderate hemophilia A when it is given either once a week, once every 2 weeks, or once every 4 weeks.

How do I take part in this clinical trial?
To be able to take part in this clinical trial, you must have been diagnosed with mild or moderate hemophilia A.

You must not have any other bleeding disorder or be taking certain treatments, and you will not be able to take part if you are pregnant or breastfeeding.

If you think this clinical trial may be suitable for you and you would like to take part, please talk to your doctor. If your doctor is of the opinion that you might be able to take part in this clinical trial, he/she may refer you to the closest clinical trial doctor. They will give you all the information you need to make your decision about taking part. You can also find the clinical trial locations on this page.

You will have some further tests to make sure you will be able to take the treatments given in this clinical trial. Some of these tests or procedures may be part of your regular medical care. They may be done even if you do not take part in the clinical trial. If you have had some of the tests recently, they may not need to be done again.

Before starting the clinical trial, you will be told about any risks and benefits of taking part in the trial. You will also be told what other treatments are available so that you may decide if you still want to take part.

If you are a woman taking part in the clinical trial, who is not currently pregnant but can become pregnant, you will either need to take contraceptive medication or not have heterosexual intercourse. This is for safety reasons.

What treatment will I be given if I join this clinical trial?
You and your doctor will decide how often you should have emicizumab treatments. Everyone who joins this clinical trial will then be assigned to one of the following 3 groups based on this decision:

Group 1

- emicizumab given as an injection under your skin every week for 4 weeks then continue with a lower dose of emicizumab every week

Group 2

- emicizumab given as an injection under your skin every week for 4 weeks then continue with the same dose of emicizumab every 2 weeks

Group 3

- emicizumab given as an injection under your skin every week for 4 weeks then continue with a higher dose of emicizumab every 4 weeks

You will not be allowed to change between groups during the first year of the study.

How often will I be seen in follow-up appointments and for how long?
During the study, you will be seen by the clinical trial doctor every 4 weeks for at least one year. These hospital visits will include regular checks and blood draws to see how you are responding to the treatment and any side effects that you may be having. You will also be given questionnaires to complete at some of your visits. Between these visits you or your caregiver will be asked to provide information on your bleeds and bleed treatments on a weekly basis. You are free to stop this treatment at any time. Your healthcare team will discuss any ongoing treatment with you.

What happens if I am unable to take part in this clinical trial?
If this clinical trial is not suitable for you, you will not be able to take part. Your doctor may suggest other clinical trials that you may be able to take part in or other treatments that you can be given. You will not lose access to any of your regular care.

For more information about this clinical trial see the **For Expert** tab on the specific For Patient page or follow this link to ClinicalTrials.gov: https://clinicaltrials.gov/ct2/show/NCT04158648

Trial-identifier: NCT04158648

Inclusion Criteria:

- Rozpoznanie #agodnej (poziom FVIII od >5% do <40%) lub umiarkowanej (poziom FVIII od #1% do #5%) wrodzonej hemofilii A bez inhibitorów FVIII z konieczno#ci# stosowania profilaktyki w oparciu o ocen# badacza
- Ujemny wynik badania w kierunku inhibitorów (tj. <0,6 BU/ml) w okresie 8 tygodni przed w##czeniem do badania
- Brak udokumentowanego inhibitora (tj. <0,6 BU/ml), okres pó#trwania FVIII <6 godzin lub odzysk FVIII<66% w okresie ostatnich 5 lat
- Udokumentowanie szczegó#ów profilaktycznego lub epizodycznego leczenia z u#yciem FVIII oraz szereg epizodów krwawienia w okresie co najmniej ostatnich 24 tygodni przed w##czeniem do badania

- Odpowiednia czynno## w#troby, nerek oraz uk#adu krwiotwórczego zgodnie z wymogami okre#lonymi w protokole

Exclusion Criteria:

- Odziedziczone lub nabyte zaburzenia krwawienia inne ni# #agodna (poziom FVIII od >5% do <40%) lub umiarkowana (poziom FVIII od #1% do #5%) wrodzona hemofilia A
- Wcze#niejsze (w okresie ostatnich 12 miesi#cy) lub aktualne leczenie choroby zakrzepowo-zatorowej (z wyj#tkiem wcze#niejszej zakrzepicy zwi#zanej z cewnikiem, która nie wymaga obecnie leczenia przeciwzakrzepowego) lub objawy choroby zakrzepowo-zatorowej
- Inne stany medyczne (np. niektóre choroby autoimmunologiczne), które mog# obecnie zwi#ksza# ryzyko krwawienia lub zakrzepicy oraz zaplanowany zabieg chirurgiczny w fazie podania dawki nasycaj#cej emicyzumabu
- Klinicznie istotna nadwra#liwo## zwi#zana z terapi# przeciwcia#ami monoklonalnymi lub sk#adnikami wstrzykiwanego preparatu emicyzumabu w wywiadzie
- Stwierdzone zaka#enie wirusem HIV z liczb# limfocytów CD4 <200 komórek/ml