

Hemophilia A Moderate Hemophilia A Severe Hemophilia A

## A clinical trial to investigate the impact of treatment with emicizumab on overall health, physical activity, and joint health in people with moderate or severe haemophilia A

A Study to Evaluate Overall Health, Physical Activity, and Joint Outcomes in Participants With Severe or Moderate Hemophilia A Without Factor VIII Inhibitors on Emicizumab Prophylaxis

<b>Trial Status</b> Active, not recruiting	<b>Trial Runs In</b> 12 Countries	<b>Trial Identifier</b> NCT05181618 2020-005092-13, 2023-505747-40-00, ISRCTN10101701 MO42623
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The source of the below information is the publicly available website [ClinicalTrials.gov](https://clinicaltrials.gov). It has been summarised and edited into simpler language.

### ***Trial Summary:***

Study MO42623 is a Phase IV, multicenter, open-label, three cohort study designed to evaluate the impact of emicizumab prophylaxis on overall health, physical activity, and joint outcomes in participants aged  $\geq 13$  and  $< 70$  years with severe hemophilia A without factor VIII (FVIII) inhibitors or moderate hemophilia A without FVIII inhibitors who are receiving FVIII prophylaxis and who will start emicizumab treatment as part of this study.

<b>Hoffmann-La Roche</b> Sponsor	<b>Phase 4</b> Phase
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Trial Identifiers

### ***Eligibility Criteria:***

<b>Gender</b> All	<b>Age</b> $\geq 13$ Years & $\leq 69$ Years	<b>Healthy Volunteers</b> No
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#### **1. Why is the BEYOND ABR clinical trial needed?**

Haemophilia A is a genetic disease caused by a missing or defective blood clotting protein called factor VIII (FVIII). People with haemophilia A bleed for longer than people without haemophilia and can also have spontaneous bleeding inside their joints (e.g. knees,

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elbows, ankles), muscles and other soft tissues (such as fat, tendons or ligaments), and difficulties with physical activities. The prevention of joint bleeds, and maintaining good joint health, is a key goal in the treatment of haemophilia.

Treatments for haemophilia A focus on replacing the missing clotting protein, FVIII, so a person can form a clot and prevent or reduce the bleeds associated with the disorder. FVIII replacement therapy is used for routine preventative treatment (known as prophylaxis). However, FVIII needs to be frequently self-administered (by the patient or care partner) directly into a vein. Also, a person's immune system can develop antibodies that stop the FVIII replacement therapy working (these are known as FVIII inhibitors).

Emicizumab is a non-factor therapy, which is administered directly under the skin (subcutaneously), and as infrequently as once every four weeks. It is approved for the treatment of haemophilia A in people with or without FVIII inhibitors, and in all age groups. It is used for routine prophylaxis in haemophilia A to prevent or reduce the number of bleeding episodes. Researchers hope that this clinical trial will provide a better understanding of any benefits of emicizumab beyond bleed control, in terms of joint health, overall health, and physical activity.

## **2. How does the BEYOND ABR clinical trial work?**

This clinical trial is recruiting people who have moderate or severe haemophilia A and are currently receiving FVIII prophylactic treatment.

The purpose of this clinical trial is to investigate the effects, good or bad, of emicizumab in people with haemophilia A. Participants who take part in this clinical trial will be given a choice of receiving one of three different dosing schedules of emicizumab: either 1.5mg/kg once a week, 3mg/kg once every two weeks, or 6mg/kg once every four weeks. These dosing schedules all provide the same dose over time, but the lower doses are given more often than the higher doses.

Participants will be given the clinical trial treatment, emicizumab, for roughly three years. Ongoing physical activity will be measured using a wearable activity tracker. Participants will be seen by the clinical trial doctor before they begin the trial and a total of nine times during the treatment period (at Months 1, 3, 6, 9, 12, 18, 24, 30, and 36). Visits in Months 9, 18, and 30 may be carried out remotely. These visits will include checks to see how the participant is responding to the treatment and any side effects they may be having. These checks may include:

# Joint magnetic resonance imaging (MRI) scans

# Joint ultrasound scans

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- # Physical examination of joints
- # Questionnaires (e.g. about quality of life, and physical activity levels)
- # Blood sample collection
- # Side effects

Participants' total time in the clinical trial will be roughly three years. Participants may be asked to come to the clinic for a safety follow-up visit 24 weeks after the last dose of emicizumab. Participants are free to stop trial treatment and leave the clinical trial at any time.

### **3. What are the main endpoints of the BEYOND ABR clinical trial?**

The main clinical trial endpoints (the main results that are measured in the trial to see if the medicine has worked) are to evaluate joint health, bleeding, quality of life, and physical activity over time as assessed by certain clinical criteria and questionnaires.

The other clinical trial endpoints include: the frequency of other medications taken by the participant to manage pain and improve mobility while taking the trial treatment, the number of bleeds related to taking part in physical activities, and the number and seriousness of any unexpected medical problems that occur while on treatment (known as adverse events).

### **4. Who can take part in this clinical trial?**

People can take part in this trial if they are aged between 13#69 years old, have been diagnosed with moderate or severe haemophilia A, are receiving FVIII prophylactic treatment, and have not developed FVIII inhibitors.

People may not be able to take part in this trial if they have previously received emicizumab prophylaxis, have certain other medical conditions, have previously received certain treatments, are pregnant or breastfeeding, or are planning to become pregnant.

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

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This is an open-label trial, which means everyone involved, including the participants and the doctors, know which medicine is being used. During the first month, all participants will be given a medium dose of emicizumab (3mg/kg) as a subcutaneous injection (beneath the surface of the skin) once a week for four weeks. After four weeks, participants will be given a choice of the following treatment schedules, which all provide the same dose of emicizumab over time but have differences in how often they are given:

# A **lower dose** of emicizumab (1.5mg/kg), given as a subcutaneous injection once **every week**

# OR, a **medium dose** of emicizumab (3mg/kg), given as a subcutaneous injection once **every two weeks**

# OR, a **higher dose** of emicizumab (6mg/kg), given as a subcutaneous injection once **every four weeks**

After 24 weeks on emicizumab, participants with unsatisfactory bleed control may be offered the option to increase their dose to 3mg/kg once every week.

The treatment schedule will be chosen in consultation with the clinical trial doctor and the dose may require more than one injection. Participants are able to change treatment schedules during the trial following discussion with the trial doctor.

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

The safety or effectiveness of the experimental treatment or use may not be fully known at the time of the trial. Most trials involve some risks to the participant, although it may not be greater than the risks related to routine medical care or the natural progression of the health condition. Potential participants will be told about any 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. These will all be described in an informed consent document (a document that provides people with the information they need to make a decision to volunteer for a clinical trial). A potential participant should also discuss these with members of the research team and with their usual healthcare provider. Anyone interested

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in taking part in a clinical trial should know as much as possible about the trial and feel comfortable asking the research team any questions about the trial.

## **Risks associated with the BEYOND ABR clinical trial drug**

Participants may have side effects (an unwanted effect of a drug or medical treatment) from the drug used in this clinical trial. Side effects can be mild to severe and even life-threatening, and can vary from person to person.

### **Emicizumab**

Potential participants will be told about the known side effects of emicizumab, and where relevant, also potential side effects based on human and laboratory studies or knowledge of similar drugs.

Emicizumab will be given as a subcutaneous injection (underneath the surface of the skin). Participants will be told about any known side effects of subcutaneous injection.

## **Potential benefits associated with the clinical trial**

Participants' health may or may not improve from participation in the clinical trial, but the information that is collected may help other people who have a similar medical condition in the future.

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For more information about this clinical trial see the **For Expert** tab on the specific ForPatients page or follow this link to ClinicalTrials.gov <https://clinicaltrials.gov/ct2/show/NCT05181618>