ForPatients
by Roche

Facioscapulohumeral Muscular Dystrophy (FSHD)

A clinical trial to compare RO7204239 with placebo in people with facioscapulohumeral muscular dystrophy

A Study to Evaluate RO7204239 in Participants With Facioscapulohumeral Muscular Dystrophy

Trial Status	Trial Runs In	Trial Identifier
Active, not recruiting	4 Countries	NCT05548556 BN43703

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

Trial Summary:

The purpose of this study is to evaluate the pharmacodynamics, safety, tolerability, pharmacokinetics, and efficacy of RO7204239, a humanized monoclonal antibody that binds to human latent myostatin, in ambulant adult participants with facioscapulohumeral muscular dystrophy (FSHD).

Hoffmann-La Roche	Phase 2
Sponsor	Phase

NCT05548556 BN43703
Trial Identifiers

Eligibility Criteria:

Gender	Age	Healthy Volunteers
All	>=18 Years & <= 65 Years	No

1. Why is the MANOEUVRE clinical trial needed?

Facioscapulohumeral muscular dystrophy, or FSHD, is a rare genetic muscle disorder. It causes weakness of the muscles in the face, shoulders, upper arms, and lower legs. The condition gets worse slowly over time and can lead to weakness in other parts of the body. Currently, there are no approved drugs for FSHD, and the disease is managed with supportive therapy such as exercise, maintaining bone health, pain management, or surgery. Researchers are looking for other ways to help people with FSHD by studying experimental drugs that may improve the functioning of the muscles.

Myostatin is a protein that the body produces naturally to control muscle growth. RO7204239 is an experimental drug that has been shown to block myostatin activity. Researchers hypothesize that experimental drugs, like RO7204239, may be an effective way to increase muscle size and strength in people with FSHD.

2. How does this clinical trial work?

This clinical trial is recruiting people who have FSHD. People can take part if they have a confirmed diagnosis of FSHD and are able to walk independently.

The purpose of this clinical trial is to compare the effects, good or bad, of RO7204239 against placebo in people with FSHD. Participants who take part in this clinical trial will receive either RO7204239 or placebo for 52 weeks, and then participants will have the option to take part in the Active Treatment Extension phase of the study, where all participants will receive RO7204239 for 52 weeks. Participants will be seen by the clinical trial doctor at least every 4 weeks. These hospital visits will include checks to see how the participant is responding to the treatment and any side effects they may be having. After a participant receives the last dose of RO7204239 in the study, there is a safety follow-up period of 6 months. Participants' total time in the clinical trial will be roughly 2.5 years. Participants are free to stop trial treatment and leave the clinical trial at any time.

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

The main clinical trial endpoints (the main results that are measured in the clinical trial to see if the medicine has worked) assess:

How RO7204239 works in the body compared with placebo, as measured using MRI scans of the thigh muscles after 52 weeks of treatment

The safety of RO7204239 compared with placebo, by recording any side effects experienced by the participants and performing blood tests, electrocardiogram, and echocardiogram

The other clinical trial endpoints assess:

Other effects of RO7204239 in the body by looking at the results of MRI scans of different muscles

What the body does to the RO7204239 once it enters the body

How RO7204239 affects the immune system

4. Who can take part in this clinical trial?

People can take part in this clinical trial if they are aged between 18 to 65 years old, have

genetic confirmation that they have FSHD (which can be assessed during the screening visit), and:

Have symptoms consistent with FSHD according to the clinical trial doctor's judgement

Are able to walk independently

Agree to maintain the same level of physiotherapy, occupational therapy, and other forms of exercise during the clinical trial

People may not be able to take part in this clinical trial if they:

Are pregnant or breastfeeding, or are planning to become pregnant

Are currently or have previously received anti-myostatin therapy

Have had treatment within 90 days with another clinical trial treatment

Have any reason (medical or otherwise) that they could not have an MRI scan

Have evidence of a heart problem

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

Everyone who joins this clinical trial will be split into two groups randomly (like flipping a coin) and given either:

RO7204239, as a subcutaneous injection (just under the skin) in the abdomen every four weeks for 52 weeks

OR placebo, as a subcutaneous injection in the abdomen every four weeks for 52 weeks

Participants will have an equal chance (1 in 2) of being placed in either group.

This is a ‘placebo-controlled’ clinical trial, which means that one of the groups will be given a substance with no active ingredients (also known as a ‘placebo’); it looks like the drug being tested. Comparing results from the different groups helps the researchers know whether any changes seen are a result of the drug or occurring by chance.

This is a double-blinded trial, which means that neither the participant nor the clinical trial doctor can choose or know the group the participant is in, until the trial is over. This approach helps to prevent bias and expectations about what will happen. However, the participant’s clinical trial doctor can find out which group the participant is in, if their safety is at risk.

After 52 weeks (1 year) in the trial, participants will have the option to take part in the Active Treatment Extension, where all participants will receive RO7204239 for 52 weeks.

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

RO7204239

Potential participants will be told about the known side effects of RO7204239, and where relevant, also potential side effects based on human and laboratory studies or knowledge of similar drugs.

RO7204239 will be given by subcutaneous injection (just under the skin) in the abdomen. Participants will be told about any known side effects of subcutaneous injections.

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.

For more information about this clinical trial see the **'For Expert'** tab on **the 'For Patients'** page or follow this link to ClinicalTrials.gov https://clinicaltrials.gov/ct2/show/NCT05548556?term=RO7204239&draw=2&rank=1