ForPatients
by Roche

Relapsing Multiple Sclerosis (RMS)

A study to see how fenebrutinib is processed in the body, how well it works to prevent signs of relapsing multiple sclerosis and how safe it is in children and teenagers with relapsing multiple sclerosis

A Pharmacokinetics (PK), Pharmacodynamics (PD), Safety and Tolerability Study of Fenebrutinib in Children and Adolescents With Relapsing Multiple Sclerosis (RMS)

Trial Status	Trial Runs In	Trial Identifier
Recruiting	7 Countries	NCT07161258 2024-519800-28-00 CN45847

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

Trial Summary:

This open label, single arm study will evaluate the PK and PD effects of fenebrutinib in children and adolescents with RMS aged between 10 and < 18 years. This study consists of a Dose Exploration Period and an Optional Extension Period. Eligible participants may choose to continue treatment with fenebrutinib in the optional extension period after completing the dose exploration period.

Hoffmann-La Roche Sponsor	**Phase 2** Phase

NCT07161258 2024-519800-28-00 CN45847
Trial Identifiers

Eligibility Criteria:

Gender	Age	Healthy Volunteers
All	>=10 Years & <= 17 Years	No

1. Why is this study needed?

Multiple sclerosis (MS) is a health condition in which the body's natural defence (the immune system) attacks the protective covering of nerve fibres in the brain and spinal cord. This leads to communication problems between the brain and the rest of the body. In relapsing multiple sclerosis (RMS), the signs or symptoms of MS return after they have improved for a while. This is known as a 'relapse'. This can cause physical impairment and

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disability, and gradual loss of brain functions like thinking, remembering, and reasoning to the point where it interferes with daily activities. New drugs which help to prevent disease worsening are needed.

This study is testing a medicine called fenebrutinib, you say this as ‘feh-NEH-broo-tuh-nib’. It is being developed to treat RMS. Fenebrutinib is an experimental medicine. This means health authorities (like the U.S. Food and Drug Administration and European Medicines Agency) have not approved fenebrutinib for the treatment of RMS.

This study aims to understand what happens to fenebrutinib once it is in the body, to see how well it works to prevent signs of RMS in the brain, and to test how safe fenebrutinib is (at different doses), in children and teenagers. This study will help determine the right dose of fenebrutinib to be used for children and teenagers with MS in possible future studies.

2. Who can take part in the study?

Children and teenagers that are 10 to 17 years of age with RMS can take part in the study if they have been given all recommended childhood vaccinations. They can have an Expanded Disability Status Scale (EDSS) score anywhere between 0 and 5.5. The EDSS measures changes in a person’s disability level over time. People with an EDSS score of 5.5 or less can walk 100 metres without the use of walking aids or needing to rest.

Children and teenagers who take part in the study must also have had one or more of the following:

- 2 relapses in the last 2 years, OR
- 1 relapse in the last year, OR
- 1 area of inflammation in the brain – known as an ‘active lesion’, visible on a magnetic resonance imaging (MRI) scan, within the past 6 months

Children and teenagers may not be able to take part in this study if they have a type of MS called ‘primary progressive MS’ or ‘non-active secondary progressive MS’. Those who have had certain treatments before, including fenebrutinib or similar medicines, or certain infections, a history of cancer, or other conditions including a disease of the brain or spinal cord cannot take part. People who are pregnant, or currently breastfeeding cannot take part in the study.

3. How does this study work?

Children and teenagers will be screened to check if they are able to participate in the study. The screening period will take place from 1 month before the start of treatment.

Everyone who joins this study will be given fenebrutinib as tablets (to be swallowed) every day. After 3 months of treatment, the study doctor and participant will decide together

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whether to continue in the next part of the study - known as the ‘optional extension’. The optional extension part of the study lasts almost 2 years.

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 first 3 months of the study, the study doctor will see participants every 2 weeks. They will check if there are any unwanted effects participants may have. Participants who continue to take fenebrutinib in the optional extension part of the study will see the study doctor every 2 weeks for the first 2 months, then again 1 month later, and then every 3 months for the remainder of the study. If participants stop taking fenebrutinib for any reason throughout any part of the study, they will have a follow-up visit 1 month after their last dose of study treatment. During this visit, the study doctor will check on the participant’s wellbeing. Total time of participation in the study will be about 3 months, and up to about 2 years if they continue in the study during the optional extension. Participants have the right to stop study treatment and leave the study at any time, if they wish to do so.

4. What are the main results measured in this study?

The main results measured in the study are:

- How fenebrutinib gets to different parts of the body and how the body gets rid of it
- The total number of new lesions seen on magnetic resonance imaging (MRI) scans of the brain after 3 months of starting study treatment

Other key results measured in the study include:

- 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 children and teenagers 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. Children and teenagers who, together with their adult guardians or caregivers, are interested in taking part in the study 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.

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Risks associated with fenebrutinib Participants may have unwanted effects of the drug 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.

Participants will be told about the known unwanted effects of fenebrutinib, and possible unwanted effects based on human and laboratory studies or knowledge of similar medicines. Known unwanted effects include higher levels of liver proteins (called ‘ALT’ and ‘AST’) in the blood than usual, which could indicate potential liver damage.

The study medicine(s) may be harmful to an unborn baby. All participants who may be (or become) capable of child-bearing, must take precautions to avoid exposing an unborn baby to the study treatment.

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