Primary Mitochondrial Myopathy Study

What is this study about?

REN001 is a new drug currently under development. To date, 106 people have been given REN001 in four clinical studies. In all these clinical studies, REN001 was considered safe and well tolerated Laboratory and some clinical study data have indicated that REN001 may improve energy production within muscle cells and may improve muscle function.

This study will determine how well REN001 works and whether it is safe and well tolerated in patients with PMM.

Who can take part?

PMM patients aged 18 years and over, men or women, with a confirmed PMM diagnosis due to a known genetic mutation or other mitochondrial DNA defect.

Participants must not be involved in any other ongoing clinical study research.

There are certain circumstances where some people might not be able to participate in this study, so the research team will carry out thorough checks (screening) to make sure patients are eligible.

What drug is being tested?

The study drug used in this study is called REN001.

The study will be made up of 2 different study treatments, REN001 100mg or placebo (dummy inactive medication). There will be a 50/50 chance of receiving REN001 100 mg study 'active' treatment.

If you agree to take part in this study, you will be asked to take the study drug (two capsules) once a day with food for 24 weeks.

What's involved in taking part?

The study will have 8 visits however only 4 of these will need to be at the study centre, the remaining 4 visits may be conducted at your home by a qualified nurse if you prefer.

- Visit 1: Screening visit (approximately 6 hours at study centre)
- Visit 2: Baseline visit (approximately 8 hours at study centre). If appropriate this visit may be conducted over 2 days due to the number of tests
- Visit 3: Week 2 (approximately 1 hour at study centre or at home)
- Visit 4: Week 4 (approximately 1.5 hours at study centre or at home)
- Visit 5: Week 12 (approximately 8 hours at study centre)
- Visit 6: Week 18 (approximately 1.5 hours at study centre or at home)
- Visit 7: Week 24 visit (approximately 8 hours at study centre)
- Visit 8: Follow Up visit (approximately 1 hour at study centre or at home)

Most visits will include blood tests, questionnaires and exercise tests (a walk test and a 30 second sit to stand test). These tests will be done to determine whether REN001 is safe and effective.

Will there be any costs associated with my participation in the study?

Patients participating in the study will be supported with the option of home nursing visits for some study visits if they would prefer not to travel to the study centre. Also, to ease the burden of travelling to and from visits, there will be a specialist company who will book and organise payment for travel and hotels for you and who will also ensure you are promptly reimbursed for any other study expenses e.g. refreshments.

Where is the study taking place?

The study is funded by Reneo Pharma Ltd and is being run at Metabolic and Genetics in Calgary (M.A.G.I.C.), #215, 971 64 AVE NE, Calgary, T2E 7Z4 in Alberta, Canada.

Are there any risks?

The study involves a series of standard clinical tests that are routinely carried out in clinical practice. This includes blood samples and exercise tests. Taking blood samples may cause some discomfort or bruising and can cause some people to faint. All of the study and exercise tests are safe and routinely used in the clinics.

REN001 has been given to healthy volunteers, to obese patients and to patients with PMM in previous clinical studies. REN001 was considered to be safe and well tolerated in all these trials) . In the PMM patient study, the most common side effects reported were:

- constipation in 4 in 23 participants and
- headache in 4 in 23participants

However, as with any treatment, side effects that have not happened before may occur.

Who will benefit?

Individuals may not benefit directly from taking this investigative drug, however we hope that the information that we collect from this study will help us understand whether this drug may be useful in treating the muscle symptoms of mitochondrial disease. The results may also be useful in understanding other muscle diseases where muscle tissue is lost or not working properly.

How do I find out more?

If you think you might be eligible and are interested in taking part in this research, or would like any further information, please contact the study team directly:

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