RIM4DMD

Rimeporide in patients with Duchenne muscular dystrophy

Hub Summary

Patients with DMD have imbalanced levels of calcium and sodium in their muscle cells, this is thought to play a key part in the damage which occurs to muscles. This study is evaluating the safety and tolerability of rimeporide. Rimeporide is a drug which works by inhibiting the movement of sodium and calcium from muscle cells. Inhibition of this mechanism has been proven to be efficient in preventing inflammation and fibrosis (muscle damage) in animal models. In addition to the preventative effect on muscle damage, rimeporide was also shown to be cardioprotective.

This investigational treatment could have no restriction on age and is not mutation specific, meaning it could treat all patients with DMD. This phase 1b study will examine Rimeporide in patients aged 6 to 14 years.

Study Number: NCT02710591

Description by EspeRare Foundation

In Duchenne Muscular Dystrophy (DMD) there is an imbalance between the levels of calcium and sodium in the muscles cells which is thought to be important in the damage which occurs overtime. Sodium/proton type 1 exchanger (NHE-1) inhibition is an innovative pathway that has proved to efficiently prevent the accumulation of muscle damage (inflammation and fibrosis) in animal models of muscular dystrophies and dilated cardiomyopathy. Based on prior safety and efficacy results in animal and humans, NHE-1 inhibition with Rimeporide represents a new therapeutic approach with no restriction on age and on genetic subtypes which could be combined to other treatments that restore or augment dystrophin. This study examines the safety and tolerability and effects on the muscles of rimeporide, in patients aged 6 to 14 years with Duchenne Muscular Dystrophy (DMD).

This study was designed as a phase Ib, multi-centre, European, open label study to evaluate the safety and tolerability and biomarkers of rimeporide, in boys aged 6 to 14 years with Duchenne Muscular Dystrophy (DMD).

Rimeporide was taken orally for 4 weeks, three times a day. With the dose adapted to the body weight of the participant. The study enrolled 20 patients with DMD, aged 6 to 14 years. 4 dose levels are being tested, in 4 different cohorts with 5 patients taking the drug at each dose level.

The study involves 6 visits to the Hospital over a maximum of 10 weeks. At each visit, patients undergo safety examinations including vital signs, physical and neurological examinations, ECG, safety and hematology, biochemistry and urinalysis, concomitant treatments review, and any symptoms and side effects review. In addition, blood samples are be withdrawn for the evaluation of Rimeporide in plasma. Finally, additional blood & urine samples are collected to explore efficacy markers. Patients also undergo 2 NMR (at screening and End of study) to develop non-invasive biomarkers for further investigations in DMD patients.

The decision to progress to the next higher dose was made after safety and tolerability data were reviewed for the preceding dose for 5 patients by SMC and determined that it is safe to proceed to the next dose level.

Primary Outcome Measures

- To evaluate the incidence, casualty and outcome of the Adverse Events [AEs] and Serious adverse event [SAEs] which might occur after multiple oral administrations of rimeporide in paediatric patients with DMD.

Secondary Outcome Measures

- To evaluate the Maximum Plasma Concentration of rimeporide in plasma in paediatric patients with DMD (Cmax)
- To evaluate the Area Under the Curve of rimeporide in plasma in paediatric patients with DMD
- To evaluate the Time to concentration peak of rimeporide in plasma in paediatric patients with DMD (Tmax)
- To evaluate the Elimination half- life of rimeporide in plasma in paediatric patients with DMD (T1 /2)

Other Outcome Measures

- To explore Pharmacodynamic biomarkers in blood to monitor muscle damages.
- Muscle composition as assessed by Magnetic Resonance Imaging (MRI).

Can I take part?

Trial Status
Trial complete

UK Locations
London - GOSH, Trial complete/terminated

Trial Sponsor
EspeRare Foundation

Phase
Ib

Length Of Participation
10 weeks

Recruitment Target
20

Ambulatory
Ambulant, Able to walk independently >75 metres

Therapeutic Category
Calcium Regulator

Age
6-14

Mutation Specific
Non-mutation specific therapies

Muscle Biopsy
No Muscle Biopsy Required

MRI
Yes

dmdhub.org

Inclusion Criteria

This trial is no longer open for recruitment.

For contact details and to find out more, please refer to dmdhub.org.

Duchenne UK

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