PepGen - CONNECT2-EDO51

A Phase 2, Randomized, Double-Blind, Placebo-Controlled, Multiple-Ascending Dose Study of PGN-EDO51 with a Long-Term Extension in Participants with Duchenne Muscular Dystrophy Amenable to Exon 51-Skipping Treatment (CONNECT2-EDO51)

Hub Summary

The CONNECT2-EDO51 Phase 2 clinical trial is a multinational, randomized, double- blind, placebo-controlled, multiple ascending dose (MAD) study, that will enroll ambulatory and non-ambulatory boys and young men living with DMD amenable to exon 51-skipping, who are at least six years of age. Participants will receive seven doses of either PGN-EDO51 or placebo at approximately four-week intervals for 24 weeks. Participants will provide a muscle biopsy at baseline and then at week 25. The trial will evaluate the safety and tolerability of PGN-EDO51 and the levels of dystrophin in skeletal muscle following repeat dosing. All participants will have the opportunity to participate in an open-label extension for 108 weeks after completing the MAD period where all participants will receive only the investigational study drug PGN-EDO51.

Study Number: EU Clinical Trial Number: 2023-508383-29

Description by PepGen

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PGN-EDO51 is an investigational exon skipping drug designed to help cells “skip” over a section (called exon 51) of the transcript that codes for dystrophin. This could allow the body to create a shortened, but functional dystrophin protein. Dystrophin can potentially stabilize or even improve muscle function.
For the first 6 months of the study, participants will be randomly assigned to receive monthly IV infusions (through a needle in a vein) of either PGN-EDO51 or placebo. The placebo will look like PGN-EDO51 but does not contain any active investigational drug.

Participants will have a 75% chance of receiving PGN-EDO51 and a 25% chance of receiving placebo during these 6 months. During the rest of the study (about 2 years), participants will receive PGN-EDO51 only (no placebo).

Participation in this clinical research study is free, and all study-related costs will be covered. You will be compensated for your study visit time and will be eligible for reasonable travel expense reimbursement.

Primary Outcome Measures

• To evaluate the safety and tolerability of PGN-EDO51 following multiple doses in male participants with Duchenne muscular dystrophy (DMD) amenable to exon 51 skipping.
• To evaluate the levels of dystrophin in skeletal muscles following multiple doses of PGN EDO51.

Secondary Outcome Measures

• To evaluate what PGN-EDO51 does to the body and what the body does to PGN-EDO51 in plasma and muscle.

Can I take part?

Inclusion Criteria

• Male by birth
• At least 6 years old
• Diagnosed with Duchenne amenable to exon 51 skipping (genetic test will be required to join)
• Weigh at least 25 kg (55 lbs)
• Willing to have a total of 2 open muscle biopsies

Exclusion Criteria

Trial Status
Trial terminated

UK Locations
Leeds, Trial complete /terminated, Oxford, Trial complete/terminated

Trial Sponsor
PepGen

Phase
2

Length Of Participation
25 weeks (plus an open label extension of 108 weeks)

Recruitment Target
24

Ambulatory
Ambulant and non-ambulant

Therapeutic Category
Exon skipping

Age
6 +

Mutation Specific
Mutation specific therapies

Muscle Biopsy
Muscle Biopsy Required

MRI
No

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•Known history or presence of any clinically significant conditions that may interfere with study safety assessments.
•Treatment with any gene replacement therapy for the treatment of DMD at any time.

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

Duchenne UK

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