

## 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

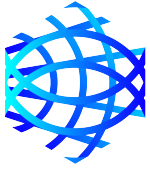
**MRI**  
No

[dmdhub.org](http://dmdhub.org)



- 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](https://dmdhub.org).



**Duchenne**  
**UK**

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