

# A Multicentre, Double-blind, Placebo-controlled, Phase 1 Study of WVE-210201 Administered Intravenously to Patients with Duchenne Muscular Dystrophy

## Hub Summary

This phase 1 study is designed to determine the safety and tolerability of Wave Life Science's Exon 51 skipping therapy.

**Study Number: NCT03508947**

## Description by Wave Life Sciences

This is a Phase 1, double-blind, placebo-controlled, single ascending dose cohort study to evaluate the safety, tolerability, and plasma concentrations of WVE-210201 in ambulatory and non-ambulatory male paediatric patients with DMD amenable to exon 51 skipping intervention.

## Primary Outcome Measures

- Number of patients with adverse events (AEs)
- Severity of AEs
- Number of patients with serious AEs (SAEs)
- Number of patients who withdraw due to AE

## Secondary Outcome Measures

- Maximum observed concentration (C<sub>max</sub>)
- Time of occurrence of C<sub>max</sub> (t<sub>max</sub>)
- Area under the plasma concentration-time curve (AUC 0-t)

## Can I take part?

### Inclusion Criteria

- Diagnosis of Duchenne muscular dystrophy (DMD) based on clinical phenotype with increased serum creatine kinase
- Documented mutation in the Dystrophin gene associated with DMD that is amenable to exon 51 skipping
- Ambulatory or non-ambulatory male patients aged ≥5 - ≤18 years
- Stable pulmonary and cardiac function as measured by: a) reproducible percent predicted forced vital capacity (FVC) ≥50% and b) left ventricular ejection fraction (LVEF) >55% in patients <10 years of age and >45% in patients ≥10 years of age, as measured (and documented) by echocardiogram within one year prior to enrolment into the study.

### Exclusion Criteria

- Severe cardiomyopathy; cardiomyopathy that is managed by angiotensin-converting enzyme (ACE) inhibitors or beta blockers is acceptable provided the patient meets the LVEF inclusion criteria.
- Need for mechanical or non-invasive ventilation OR anticipated need for mechanical or non-invasive ventilation within the next year, in the opinion of the Investigator.
- Changes in nutritional or herbal supplements or concomitant medications within 1 month prior to Screening visit or plans to modify dose or regimen during the study.
- Currently on anticoagulants or antithrombotics.
- Received treatment with eteplirsen or ataluren within the past 14 weeks.

**Trial Status**  
Trial complete



#### UK Locations

London - Evelina, Trial complete/terminated,  
London - GOSH, Trial complete/terminated,  
Alder Hey, Trial complete/terminated,  
Bristol, Trial complete/terminated



#### Trial Sponsor

Wave Life Sciences



#### Age

5 to 18 years



#### Mutation Specific

Mutation specific therapies, must be amenable to exon 51 skipping



#### Muscle Biopsy

No Muscle Biopsy Required



#### MRI

No



#### Phase

1



#### Recruitment Target

40



#### Ambulatory

Ambulant and non-ambulant



#### Therapeutic Category

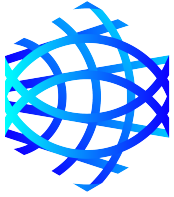
Exon skipping

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


  
DMD HUB

- Received prior treatment with drisapersen.
- Received any investigational drug within the past 3 months or 5 half-lives, whichever is longer.

For contact details and to find out more, please refer to [dmdhub.org](https://dmdhub.org).



**Duchenne**  
**UK**

PDF created on 16/05/2024.