

## 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 (Cmax)
- Time of occurrence of Cmax (tmax)
- 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.
- ✗ 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).

## 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


 **Phase**  
1

 **Recruitment Target**  
40

 **Ambulatory**  
Ambulant and non-ambulant

 **Therapeutic Category**  
Exon skipping

 **Age**  
5 to 18 years

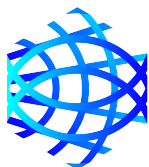
 **Mutation Specific**  
Mutation specific therapies, must be amenable to exon 51 skipping

 **Muscle Biopsy**  
No Muscle Biopsy Required

 **MRI**  
No

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DMD HUB



Duchenne  
UK

PDF created on 21/08/2025.

