Wave Life Sciences Exon 51

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.

Secondary Outcome Measures

- Maximum observed concentration (Cmax)
- Time of occurrence of Cmax (tmax)
- Area under the plasma concentration-time curve (AUC 0-t)

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

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)

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

Recruitment Target
40

Ambulatory
Ambulant and non-ambulant

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

Therapeutic Category
Exon skipping

Muscle Biopsy
No Muscle Biopsy Required

Age
5 to 18 years

MRI
No

Phase
1

>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.

Duchenne UK

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