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.

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.

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- 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.