

DYSTANCE 51 [TERMINATED]



A Randomised, Double-blind, Placebo-controlled, Efficacy and Safety Study of Suvodirsen (WVE-210201) With Open Label Extension in Ambulatory Patients With Duchenne Muscular Dystrophy

Hub Summary

Please note that Wave have stopped the development of this drug after the Phase 1 Open-label extension failed to meet its primary endpoint.

DYSTANCE 51 is a phase 2/3 clinical trial designed to evaluate the efficacy and safety of WVE-210201 (suvodirsen) in ambulant boys with DMD mutations amenable to exon 51 skipping.

DYSTANCE 51 is comprised of two phases, a placebo-controlled phase and an open-label phase. In the placebo-controlled phase, patients will be randomized to receive suvodirsen 3 mg/kg, suvodirsen 4.5 mg/kg or placebo for 48 weeks. Dystrophin protein levels and functional outcomes will be assessed for each patient throughout the initial 48-week treatment period.

Each participant will have two biopsies in total, one at baseline, and one at either week 12, 22, or 46.

Following completion of the placebo-controlled phase of the study, patients will enter the open-label phase to receive ongoing treatment with suvodirsen. There will be no placebo or biopsies in the open label phase. However, functional assessments will continue.

Study Number: NCT03907072

Description by Wave Life Sciences Ltd

DYSTANCE 51 is a global, phase 2/3, multicentre, randomised, double-blind, placebo-controlled clinical trial that will evaluate the efficacy and safety of suvodirsen in ambulatory male paediatric patients with DMD amenable to exon 51 skipping intervention.

Primary Outcome Measures

- Change from baseline in dystrophin level (% normal dystrophin) - US/other regions (as applicable)
- Change from baseline in North Star Ambulatory Assessment (NSAA) - EU/other regions (as applicable)

Secondary Outcome Measures

- Change from baseline in North Star Ambulatory Assessment (NSAA)
- Change from baseline in dystrophin level (% normal dystrophin)
- Change from baseline in upper limb proximal strength
- Change from baseline in 4-stair climb
- Change from baseline in the 10-meter walk/run test
- Change from baseline in forced vital capacity
- Change from baseline in the 95th percentile of stride velocity

Can I take part?

Inclusion Criteria

Trial Status
Trial terminated

UK Locations
London - GOSH, Trial complete/terminated, Alder Hey, Trial complete/terminated, Leeds, Trial complete/terminated, Newcastle, Trial complete/terminated, Oxford, Trial complete/terminated

Trial Sponsor
Wave Life Sciences Ltd

Age
5-12 years old

Mutation Specific
Mutation specific therapies, Amenable to exon 51 skipping

Muscle Biopsy
Muscle Biopsy Required

Phase
Phase 2/3

Length Of Participation
2 years

Recruitment Target
150

Ambulatory
Ambulant

Therapeutic Category
Exon skipping



dmdhub.org



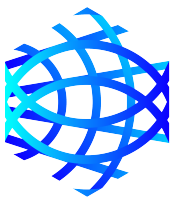
DMD HUB

- Diagnosis of 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 male, able to walk independently for at least 10 meters in 10 seconds or less at the time of Screening visit (performed as part of the NSAA)
- 5 to 12 years of age (up to 13th birthday) at the time of randomisation
- Stable pulmonary and cardiac function, as measured by:
 1. Reproducible percent predicted forced vital capacity (FVC) $\geq 50\%$
 2. 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
- Currently on a stable corticosteroid therapy regimen, defined as initiation of systemic corticosteroid therapy occurred ≥ 6 months prior to Screening, and no changes in dosing ≤ 3 months prior to Screening visit

Exclusion Criteria

- Cardiac insufficiency:
 1. Severe cardiomyopathy that, in the opinion of the Investigator, prohibits participation in this study; however, cardiomyopathy that is managed by angiotensin-converting-enzyme (ACE) inhibitors or beta blockers is acceptable provided the patient meets the LVEF inclusion criterion
 2. Any other evidence of clinically significant structural or functional heart abnormality
 3. A cardiac troponin I value > 0.2 ng/mL
- Need for daytime mechanical or non-invasive ventilation OR anticipated need for daytime mechanical or non-invasive ventilation within the next year, in the opinion of the Investigator. Nighttime non-invasive ventilation is permitted
- Received prior treatment with drisapersen or with an investigational peptide-conjugated phosphorodiamidate morpholino oligomer (PPMO)
- Received prior treatment with gene therapy for DMD
- Received treatment with ataluren or eteplirsen within the 14 weeks prior to the planned Baseline biopsy collection
- Received any investigational drug within 3 months or 5 half-lives, whichever is longer, prior to the planned Baseline biopsy collection

For contact details and to find out more, please refer to dmdhub.org.



**Duchenne
UK**