TERMINATED: DYSTANCE 51

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

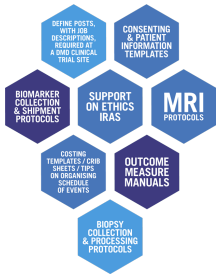
Phase
Phase 2/3

Length Of Participation
2 years

Recruitment Target
150

Ambulatory
Ambulant

Therapeutic Category
Exon skipping



dmdhub.org

- 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) ≥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 Join us to end Duchenne

PDF created on 08/04/2020.