# NS Pharma - RACER53

## A Phase 3 Randomized, Double-blind, Placebo-controlled, Multi-center Study to Assess the Efficacy and Safety of Viltolarsen in Ambulant Boys With Duchenne Muscular Dystrophy (DMD)

**Hub Summary**

This is a placebo-controlled phase 3 study, designed to investigate the efficacy and safety of NS Pharma's exon skipping drug, Viltolarsen. It will be focusing on patients with mutations amenable to exon 53 skipping and will involve a weekly intravenous infusion over 48 weeks.

The dystrophin gene has 79 pieces called exons. These link together to form a code which instructs the body to make dystrophin. If there is a fault, as in DMD, the sequence is broken and the code cannot be read. Exon skipping drugs complete the sequence and leads to a shortened dystrophin being produced that still contains the important pieces of this molecule.

**Study Number: NCT04060199**

**Description by NS Pharma**

This is a Phase 3 randomized, double-blind, placebo-controlled, multi-center study to assess the efficacy and safety of Viltolarsen in ambulant boys with Duchenne muscular dystrophy. Eligible patients with out-of-frame deletion mutations amenable to exon 53 skipping will be randomized to receive once weekly intravenous (IV) infusions of 80 mg/kg Viltolarsen or placebo for up to 48 weeks.

The study will enroll approximately 74 patients amenable to exon 53 skipping. Clinical efficacy will be assessed at regularly scheduled study visits, including functional tests such as Time to Stand Test (TTSTAND), Time to Run/Walk 10 Meters Test (TTRW), Six-minute Walk Test (6MWT), North Star Ambulatory Assessment (NSAA), Time to Climb 4 Steps Test (TTCLIMB) and Hand-held dynamometer (elbow extension, elbow flexion, knee extension and knee flexion on the dominant side only).

Safety will be assessed through the collection of adverse events (AEs), laboratory tests, electrocardiograms (ECGs), vital signs, and physical examinations throughout the study.

Blood samples will be taken periodically throughout the study to assess the pharmacokinetics of study drug.

### Primary Outcome Measures

1. TTSTAND [ Time Frame: baseline to 48 weeks of treatment ]
   Change in Time to Stand (TTSTAND)

### Secondary Outcome Measures

1. TTRW [ Time Frame: baseline to 48 weeks of treatment ]
   Change in Time to Run/Walk 10 Meters Test (TTRW)
2. 6MWT [ Time Frame: baseline to 48 weeks of treatment ]
   Change in Six-minutes Walk Test (6MWT)
3. NSAA [ Time Frame: baseline to 48 weeks of treatment ]
   Change in North Star Ambulatory Assessment (NSAA)
   The NSAA is a functional scale devised for use

## Trial Status
Trial complete

**UK Locations**
London - GOSH, Trial complete/terminated, Birmingham, Trial complete /terminated, Glasgow, Trial complete/terminated, Manchester, Trial complete /terminated

**Trial Sponsor**
NS Pharma

**Phase**
3

**Length Of Participation**
48 weeks

**Recruitment Target**
74

**Ambulatory**
Ambulant

**Therapeutic Category**
Exon Skipping

**Age**
4-7

**Mutation Specific**
Mutation specific therapies, Exon 53

**Muscle Biopsy**
No Muscle Biopsy Required

**MRI**
No

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in ambulant children with Duchenne muscular dystrophy (DMD). It consists of 17 activities graded 0 (unable to perform), 1 (performs with modifications), 2 (normal movement). It assesses abilities necessary to remain ambulant that have been found to progressively deteriorate in untreated DMD patients, as well as in other muscular dystrophies such as Becker Muscular Dystrophy. NSAA Total Score ranges from 0 to 34, with a score of 34 implying normal function.

4. TTCLIMB [ Time Frame: baseline to 48 weeks of treatment ]
   Change in Time to Climb 4 Steps Test (TTCLIMB)
5. Hand-held dynamometer
   [ Time Frame: baseline to 48 weeks of treatment ]
   The force generated for each muscle strength (elbow extension, elbow flexion, knee extension, and knee flexion on the dominant side only) will be measured by Hand-held dynamometer.

**Can I take part?**

## Inclusion Criteria

- ✔ Male  4 years and < 8 years of age
- ✔ Confirmed DMD mutation(s) in the dystrophin gene that is amenable to skipping of exon 53 to restore the dystrophin mRNA reading frame
- ✔ Able to walk independently without assistive devices
- ✔ TTSTAND < 10 seconds
- ✔ Stable dose of glucocorticoid (GC) for at least 3 months prior to study entry and is expected to remain on stable dose of GC treatment for the duration of the study
- ✔ Other inclusion criteria may apply

## Exclusion Criteria

- ✖ Current or history of chronic systemic fungal or viral infections
- ✖ Acute illness within 4 weeks prior to the first dose of study drug
- ✖ Evidence of symptomatic cardiomyopathy (Note: Asymptomatic cardiac abnormality on investigation would not be exclusionary)
- ✖ Allergy or hypersensitivity to the study drug or to any of its constituents
- ✖ Severe behavioral or cognitive problems that preclude participation in the study, in the opinion of the investigator
- ✖ Previous or ongoing medical condition, medical history, physical findings or laboratory abnormalities that could affect safety, make it unlikely that treatment and follow-up will be correctly completed or impair the assessment of study results, in the opinion of the investigator;
- ✖ Surgery within the 3 months prior to the first dose of study drug or surgery is planned for anytime during the duration of the study
- ✖ Participant has positive test results for hepatitis B antigen, hepatitis C antibody or human immunodeficiency virus (HIV)

- ✖ Currently taking any other investigational drug or has taken any other investigational drug within 3 months prior to the first dose of study drug or within 5 times the half-life of a medication, whichever is longer
- ✖ Previously enrolled in an interventional study of viltolarsen
- ✖ Currently taking any other exon skipping agent or has taken any other exon skipping agent within 3 months prior to the first dose of study drug
- ✖ Having taken any gene therapy
- ✖ Other exclusion criteria may apply

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

Duchenne UK

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