

## Study to Assess the Safety and Efficacy of Viltolarsen in Ambulant Boys With DMD (RACER53-X)

### Hub Summary

This is a Phase 3, multi-center, open-label extension study in ambulant boys with DMD who have completed the 48-week treatment period of either viltolarsen or placebo in Study NS-065/NCNP-01-301.

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: NCT04768062**

### Description by NS Pharma, Inc.

This Phase 3 study is a multi-center, open-label extension study in ambulant boys with DMD who have completed the 48-week treatment period of either viltolarsen or placebo in Study NS-065/NCNP-01-301. Patients will receive viltolarsen administered IV at weekly doses of 80 mg/kg.

Study NS-065/NCNP-01-302 will be comprised of a 96-week treatment period.

### Primary Outcome Measures

1. Number of participants with treatment related Adverse Events as assessed by CTCAE v4.03 [ Time Frame: baseline to up to 96 weeks of treatment ]

### Secondary Outcome Measures

1. Time to Stand Test (TTSTAND)  
[ Time Frame: baseline to 96 weeks of treatment ]  
Change in Time to Stand
2. Time to Run/Walk 10 Meters Test (TTRW)  
[ Time Frame: baseline to 96 weeks of treatment ]  
Change in Time to Run/Walk 10 meters
3. Six-minute Walk Test (6MWT)  
[ Time Frame: baseline to 96 weeks of treatment ]  
Change in Six-minute Walk
4. North Star Ambulatory Assessment (NSAA)  
[ Time Frame: baseline to 96 weeks of treatment ]  
Change in North Star Ambulatory Assessment

## Trial Status Enrolling by invitation

**UK Locations**  
London - GOSH, Enrolling by invitation, Birmingham, Fully recruited, Glasgow, Enrolling by invitation, Manchester, Enrolling by invitation

**Trial Sponsor**  
NS Pharma, Inc.

**Phase**  
Phase 3

**Length Of Participation**  
96-week treatment period.

**Recruitment Target**  
74

**Ambulatory**  
Ambulant

**Therapeutic Category**  
Exon Skipping

**Age**  
5-8

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

**Muscle Biopsy**  
No Muscle Biopsy Required

**MRI**  
No

[dmdhub.org](https://dmdhub.org)

5. Time to Climb 4 Stairs Test (TTCLIMB)  
[ Time Frame: baseline to 96 weeks of treatment ]

Change in Time to Climb 4 Stairs

6. Muscle Strength Measured by Hand-Held Dynamometer [ Time Frame: baseline to 96 weeks of treatment ]

Change in Muscle Strength Measured by Hand-Held Dynamometer

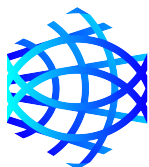
**Can I take part?**

**Inclusion Criteria**

1. Patient has completed the NS-065/NCNP-01-301 study;
2. Patient's parent(s) or legal guardian(s) has (have) provided written informed consent and Health Insurance Portability and Accountability Act authorization, where applicable, prior to any study-related procedures; patients will be asked to give written or verbal assent according to local requirements;
3. Patient and parent(s)/guardian(s) are willing and able to comply with scheduled visits, investigational product (IP) administration plan, and study procedures.

**Exclusion Criteria**

1. Patient had an adverse event in Study NS-065 /NCNP-01-301 that, in the opinion of the investigator and/or the sponsor, precludes safe use of viltolarsen for the patient in this study;
2. Patient had a treatment which was made for the purpose of dystrophin or dystrophin-related protein induction after completion of Study NS-065/NCNP-01-301;
3. Patient took any other investigational drug(s) during or after completion of Study NS-065 /NCNP-01-301;
4. Patient is judged by the investigator and/or the sponsor not to be appropriate to participate in the extension study for any reason.



Duchenne  
UK

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