NS Pharma - RACER53-X

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
Fully recruited

UK Locations
London - GOSH, Fully recruited, Birmingham, Fully recruited, Glasgow, Fully recruited, Manchester, Fully recruited

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

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

PDF created on 08/11/2026.