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

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

Patient has completed the NS-065/NCNP-01-301 study;

Trial Status
Enrolling by invitation

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

Trial Sponsor
NS Pharma, Inc.

Age
5-8

Mutation Specific
Mutation specific therapies, Exon 53

Muscle Biopsy
No Muscle Biopsy Required

MRI
No

Phase
Phase 3

Length Of Participation
96-week treatment period.

Recruitment Target
74

Ambulatory
Ambulant

Therapeutic Category
Exon Skipping

dmdhub.org

1.
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.

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

Duchenne UK