

NEWS RELEASE



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Morpholino Oligonucleotide NS-065/NCNP-01 (viltolarsen) Presentation of Phase II Study in the US

Nippon Shinyaku Co., LTD. (Nippon Shinyaku; Headquarters, Kyoto; President, Shigenobu Maekawa) announced that the presentation of an update from the US Phase II clinical study of NS-065/NCNP-01 (viltolarsen) in patients with Duchenne muscular dystrophy (DMD) was made at the 24th Annual Congress of the World Muscle Society (WMS) held in Copenhagen, Denmark. This Phase II study was conducted by NS Pharma, Inc., (Headquartered in Paramus, New Jersey, US; President, Tsugio Tanaka) which is a wholly-owned subsidiary of Nippon Shinyaku Co., Ltd.

Study Chair, Paula Clemens, M.D., of the University of Pittsburgh and the VA Pittsburgh Healthcare System in Pittsburgh, PA. presented data at WMS on the restoration of dystrophin protein in skeletal muscle and results of physical function tests and quantitative muscle strength by viltolarsen.

Participants in the trial (N=16), were boys 4 to 9 years of age with DMD. The first part of the trial consisted of 4 weeks of double-blind, placebo-controlled, randomized treatment (5 participants received placebo). This was followed by weekly intravenous infusions of viltolarsen at either 40 mg/kg or 80 mg/kg for a total of 20 to 24 weeks of viltolarsen. Upon completion of this trial, all participants continued on in the open label extension study for up to 144 weeks of treatment.

After treatment of viltolarsen for 20-24 weeks, drug-induced dystrophin protein in muscle of patients and exon 53 skipping efficiency were tested. In addition to safety and tolerability, timed motor function was assessed with time to stand, time to run/walk 10 meters velocity, time to climb four stairs velocity, 6-minute walk test distance, and North Star Ambulatory Assessment (NSAA) scores.

"The 24-week results, coupled with interim data from the open-label extension in which treatment was lengthened out to 73 weeks, can support further development of viltolarsen" said Paula Clemens, M.D.

Viltolarsen is being studied for the treatment of DMD amenable to exon 53 skipping and its new drug applications (NDA) were recently submitted. The safety and efficacy of viltolarsen has not been established by any Health Authorities. It is not been approved for the treatment of DMD in the USA or globally.

Additional information about this Phase II trial can be found on www.ClinicalTrials.gov (initial 24-week treatment period: [NCT02740972](https://clinicaltrials.gov/ct2/show/study/NCT02740972); 144-week open-label extension: [NCT03167255](https://clinicaltrials.gov/ct2/show/study/NCT03167255)).

About Duchenne muscular dystrophy

Duchenne muscular dystrophy (DMD) is a progressive form of muscular dystrophy that occurs primarily in males. DMD causes progressive weakness and loss (atrophy) of skeletal and heart muscles. Early signs of DMD may include delayed ability to sit, stand, or walk and difficulties learning to speak. DMD may also affect learning and memory, as well as communication and certain social emotional skills. Most children with DMD use a wheelchair full-time by age 13. Heart and respiratory muscle problems begin in the teen years and lead to serious, life- threatening complications.

About NS Pharma

NS Pharma, Inc., is a wholly owned subsidiary of Nippon Shinyaku Co., Ltd. For more information, please visit <http://www.nspharma.com/>.

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