# *NEWS RELEASE*

NIPPON SHINYAKU CO., LTD.

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## NS-065/NCNP-01 (Viltolarsen) of Nippon Shinyaku' in-house product
## Presentation on results of Phase II study in US/Canada

Nippon Shinyaku Co., Ltd. (Nippon Shinyaku; Headquarters, Kyoto City; President, Shigenobu Maekawa) announced today the presentation by Dr. Paula Clemens, University of Pittsburgh School of Medicine of results of a US/Canada Phase II clinical trial of viltolarsen [*1] at the 23rd International Annual Congress of the World Muscle Society held in Mendoza, Argentina. The clinical trial treated 16 boys with Duchenne muscular dystrophy (DMD) for about 24 weeks (5 participants had placebo for the first 4 weeks of treatment) with intravenous viltolarsen at either 40 mg/kg or 80 mg/kg, and was carried out by the Cooperative International Neuromuscular Research Group (CINRG) [*2].

After 20-24 weeks treatment, drug-induced dystrophin and exon 53 skipping efficiency were seen in both dose groups of viltolarsen.

Timed function tests of viltolarsen-treated patients vs. age- and treatment-matched natural history controls showed improvements for time to run/walk 10 meters velocity, time to stand from supine velocity and 6 minute walk test meters. "These preliminary findings suggest clinical improvement of DMD boys as measured by timed function tests," said Dr. Paula Clemens, Study Chair.

No TEAEs required discontinuation or dose reduction of study drug. All AEs were mild or moderate.

DMD is a debilitating and progressive muscle disease, and is caused by the loss of dystrophin protein in patient muscle. An approach to restore dystrophin expression in patient muscle is through exon skipping [*3], where nucleic acid drugs are delivered to change the RNA splicing patterns.

NS Pharma, Inc. (Headquarters, New Jersey; President, Tsugio Tanaka), a US-based subsidiary of Nippon Shinyaku Co., Ltd., is developing viltolarsen in US/Canada as a new therapy for DMD for patients with dystrophin mutations that are amenable to exon 53 skipping.

The information on the presentation is uploaded at the following website. (http://www.nspharma.com/)

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**NOTES:**

*1 Viltolarsen, an exon skipping morpholino nucleic acid drug, is being developed to treat DMD patients with mutations of the dystrophin gene that are amenable to exon 53 skipping. NS-065/NCNP-01 was discovered through collaborative research of National Center of Neurology and Psychiatry (Kodaira City, Tokyo; President, Hidehiro Mizusawa and Director, Shin'ichi Takeda).

*2 CINRG was founded in 2000 as an international academic clinical trial network, with a focus on pediatric neuromuscular disease. CINRG has enrolled over 1,500 patients into clinical research studies and clinical trials. Recent studies include the CINRG Duchenne Natural History Study (DNHS) with 440 DMD patients and over 100 healthy peers followed by expert neuromuscular physicians at approximately 25 clinical sites in 10 countries.

See www.cinrgresearch.org

[*3] Exon skipping has potential as a therapy for patients with DMD, based on the use of a synthetic, antisense oligonucleotide, to restore the amino acid reading frame by skipping certain exons of the transcription product (mRNA) to be translated into protein. This approach produces a dystrophin protein that is shorter than normal but still functional, to improve muscle function.