NEWS RELEASE

NIPPON SHINYAKU CO., LTD.

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Presentation of top line results of US/Canada Phase II study of NS-065/NCNP-01, for Duchenne muscular dystrophy

Nippon Shinyaku Co., Ltd. (Nippon Shinyaku; Headquarters, Kyoto City; President, Shigenobu Maekawa) announced today that top line results of a US/Canada Phase II clinical trial of NS-065/NCNP-01[*1] were presented at the 2018 New Directions in Biology and Disease of Skeletal Muscle Conference held in New Orleans, LA.

Dr. Paula Clemens, University of Pittsburgh and Study Chair made a presentation at the 2018 New Directions in Biology and Disease of Skeletal Muscle Conference on June 27 (CST), reporting partial restoration of dystrophin in skeletal muscle by NS-065/NCNP-01.

Duchenne muscular dystrophy (DMD) is a debilitating and progressive muscle disease, and is caused by the loss of the dystrophin protein in patient muscle. An approach to restore dystrophin expression in patient muscle is through exon skipping[*2], where nucleic acid drugs are delivered to change the RNA splicing patterns. About 8% of DMD patients have gene mutations that can be rescued by a drug targeting exon 53 for exon skipping.

NS Pharma, Inc. (Headquarters, New Jersey; President, Tsugio Tanaka), a US-based subsidiary of Nippon Shinyaku Co., Ltd., is developing NS-065/NCNP-01[*1] in US/Canada as a new therapy for DMD for patients with dystrophin mutations that are amenable to exon 53 skipping.

A 24-week dose ranging study of intravenous NS-065/NCNP-01 (clinicaltrials.gov NCT02740972) was conducted by seven Cooperative International Neuromuscular Research Group (CINRG)[*3] study sites enrolling 16 boys, age 4-10 years, equally distributed between 2 dose cohorts, 40 and 80 mg/kg/week. Drug-induced increases in dystrophin content of muscle were seen in all patients (16/16), with dystrophin rescue averaging 5.8% of normal levels (range 1.1 – 14.4%).

Neither serious adverse event (SAE) nor drug-related AE was observed in this study. All AEs were mild or moderate.

"We were encouraged to see the consistency of drug-induced dystrophin expression in all patients studied," said Dr. Paula Clemens, Study Chair, and Medical Director of the CINRG network. "Dystrophin levels driven by NS-065/NCNP-01 were higher than other clinical trials of exon skipping in DMD reported to date."

"The robust changes in NS-065/NCNP-01-induced dystrophin expression may have clinical benefits for DMD patients," said Dr. Eric Hoffman, Vice President of AGADA BioSciences, and Professor of Pharmaceutical Sciences, Binghamton University – SUNY.

The data presented at the meeting are uploaded at NS Pharma's website.
(http://www.nspharma.com/)

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NOTES:

*1 NS-065/NCNP-01, 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 a collaborative research of National Center of Neurology and Psychiatry (Kodaira City, Tokyo; President, Hidehiro Mizusawa and Corporate Director, Shin'ichi Takeda)

[*2]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.

[*3]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