NEWS RELEASE

NIPPON SHINYAKU CO., LTD.

October 3, 2018

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

Nippon Shinyaku Co., Ltd. (Nippon Shinyaku; Headquarters, Kyoto City; President, Shigenobu Maekawa) announced today that the results of a Phase I/II clinical trial of viltolarsen[*1] in Japan were presented at The 23rd International Annual Congress of the World Muscle Society held in Mendoza, Argentina.

Hirofumi Komaki, MD, PhD, Study Chair, and Director general of Translational Medical Center, National Center of Neurology and Psychiatry (NCNP) made a presentation, reporting the restoration of dystrophin protein in skeletal muscle and results of physical function tests by viltolarsen.

A 24-week dose finding study of intravenous viltolarsen (Japic CTI-163291) was conducted in 16 boys, age 5-12 years suffering from Duchenne muscular dystrophy (DMD), distributed between 2 dose cohorts, 40mg/kg and 80mg/kg. Safety and effectiveness was evaluated in this study.

No adverse events required discontinuation. All adverse events were mild or moderate in this study. Increases in Exon 53 skipping efficiency were observed in all patients and drug-induced increases in dystrophin content of muscle were seen in 14 out of 16 patients.

Although this trial is conducted by limited numbers of subjects, in the correlation analysis between dystrophin expression and physical functions as a secondary endpoint, dependency of the reduction of physical functions' worsening was seen according to dystrophin expression.

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.

Nippon Shinyaku is developing viltolarsen in Japan 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/)

Media contacts:

NS Pharma, Inc. (Paramus, NJ)
http://www.nspharma.com/
Tel: +1-201-986-3860,
Fax: +1-201-986-3865,
E-mail: info@nspharma.com

Nippon Shinyaku Co., Ltd. (Kyoto, Japan)
http://www.nippon-shinyaku.co.jp/english/
Tel: +81-75-321-9103
Fax: +81-75-321-9128
E-mail: kouhou@mb.nippon-shinyaku.co.jp

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 a collaborative research of NCNP (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 oligonucleotide, known as an 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.