NEWS RELEASE

NIPPON SHINYAKU CO., LTD.

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NS-065/NCNP-01 (viltolarsen) under development, granted Orphan Drug Designation by MHLW

Nippon Shinyaku Co., Ltd. (Nippon Shinyaku; Headquarters, Kyoto; President, Shigenobu Maekawa) today announced that NS-065/NCNP-01 (generic name: viltolarsen) under development was granted orphan drug designation by the Ministry of Health, Labor and Welfare (MHLW) for an expected indication of Duchenne muscular dystrophy amenable to exon 53 skipping therapy and from which certain dystrophin gene is deleted.

Duchenne muscular dystrophy (DMD) is an inherited severe muscle disorder with the highest incidence that is limited to male children. It causes a loss of muscle power due to a deficiency of normal dystrophin, a protein involved in constructing the framework of muscle cells. Because there is no effective treatment for DMD other than steroids, the development of an effective new treatment is desired. Viltolarsen is a drug candidate which is expected to be effective for DMD amenable to dystrophin exon 53 skipping.

Orphan drug is defined as the medicine of which medical needs are particularly high for a disease of which patient number is less than 50,000 in Japan. Orphan drug designation may shorten the regulatory approval by several months in Japan. Viltolarsen was registered as "SAKIGAKE designation" of MHLW in October, 2015.

Nippon Shinyaku has been working actively having a sense of mission to develop agents for the treatment of intractable and rare diseases, and aiming at providing medicine for DMD patients as soon as possible.

< NS-065/NCNP-01 (viltolarsen)>

NS-065/NCNP-01 is a morpholino antisense oligonucleotide, which was co-discovered by Nippon Shinyaku and National Center of Neurology and Psychiatry (NCNP: Kodaira City, Tokyo; President, Hidehiro Mizusawa, Executive Director, Shin'ichi Takeda). A phase 1/2 study and phase 2 study was carried out in Japan and the US respectively. The clinical study in the US was conducted by NS Pharma, Inc. (Headquarters, New Jersey, US; President, Tsugio Tanaka) which is a subsidiary of Nippon Shinyaku and initiated a rolling submission of NDA to FDA. Viltolarsen was granted Fast Track designation, Orphan Disease designation, and Rare Pediatric Orphan designation by US FDA.

<SAKIGAKE designation> (Japanese version of Breakthrough Therapy designation)

The designation is to promote R&D in Japan aiming at early practical application for world's first, domestically-produced and innovative pharmaceutical products for serious and life-threatening diseases. This is aiming to shorten reviewing time for approval, facilitating a prioritized consultation and review for regulatory approval.

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