NEWS RELEASE

NIPPON SHINYAKU CO., LTD.

November 6, 2019

Designation of Oligonucleotides NS-065/NCNP-01(viltolarsen)
Subjected to Conditional Early Approval System

Nippon Shinyaku Co., Ltd. (Nippon Shinyaku; Headquarters, Kyoto; President, Shigenobu Maekawa) announced that oligonucleotides NS-065/NCNP-01 (generic name: viltolarsen) was designated as a drug candidate subjected to conditional early approval system* by the Ministry of Health, Labor and Welfare (MHLW).

Viltolarsen is an antisense oligonucleotide which was submitted regulatory approval in Japan in September this year as an expected indication of Duchenne muscular dystrophy (DMD) amenable to dystrophin exon 53 skipping. DMD causes a deficiency of dystrophin protein in muscle cells, which is a progressive genetic disorder. Since there is no effective treatment for DMD other than steroids as supportive care, the development of an effective new treatment according the cause is desired.

Viltolarsen was registered as "SAKIGAKE designation" of MHLW in October, 2015 and designated as an orphan disease in August, 2019.

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.

* Conditional Early Approval System

"Conditional Early Approval System" is a system to put highly useful and effective drugs for treating serious diseases into practical use as early as possible. The system must be subjected to the following 4 conditions.

1. For severe diseases
2. Apparent improvement of medical care
3. Confirmatory clinical trials don't have sufficient feasibility.
4. Confirmation of a certain degree of efficacy and safety through clinical trials other than confirmatory clinical trials.

Please refer to the following URL in details.
https://www.mhlw.go.jp/file/05-Shingikai-10601000-Daijinkanboukouseikagakuka-Kouseikagakuka/0000184962.pdf

< 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.

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