NEWS RELEASE

NIPPON SHINYAKU CO., LTD.

October 2, 2019

U.S. FDA Submission of New Drug Application for NS-065/NCNP-01 (viltolarsen)

Nippon Shinyaku Co., Ltd. (Nippon Shinyaku; Headquarters, Kyoto; President, Shigenobu Maekawa) announced that it has completed the submission of its rolling New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) for NS-065/NCNP-01 (viltolarsen), which is under development for the treatment of Duchenne muscular dystrophy (DMD) amenable to exon 53 skipping.

DMD is an inherited muscle disorder that male children develop. 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 patients with DMD amenable to exon 53 skipping other than steroids, it is expected that this would be the first effective new treatment for this population.

Viltolarsen is a drug candidate which is expected to generate functional dystrophin proteins and be effective for DMD amenable to dystrophin exon 53 skipping.

Viltolarsen was granted a Rare Pediatric Disease Designation, Orphan Drug Designation, and Fast Track Designation in the U.S., and "SAKIGAKE designation" and “Orphan drug designation” in Japan. Nippon Shinyaku has announced that it has submitted its NDA to the Ministry of Health, Labour and Welfare on September 26, 2019.

The clinical study in the US was conducted by NS Pharma, Inc. (Headquartered in Paramus, New Jersey, US; President, Tsugio Tanaka) which is a wholly-owned subsidiary of Nippon Shinyaku Co., Ltd. Following the completion of their rolling NDA submission, Nippon Shinyaku and NS Pharma will work with the FDA to confirm that the application meets the requirements for review.

Nippon Shinyaku's mission is to work actively to develop agents for the treatment of intractable and rare diseases and provide treatment for DMD patients as soon as possible.

Contact

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