

NEWS RELEASE



February 7, 2019

NDA Accepted for Filing by the FDA for Antisense Oligonucleotide Viltolarsen (NS-065/NCNP-01)

KYOTO, Japan and PARAMUS, NJ: February 7, 2020 - Nippon Shinyaku Co., Ltd. (President, Shigenobu Maekawa) and NS Pharma, Inc. (President, Tsugio Tanaka), a wholly owned subsidiary of Nippon Shinyaku, announced today that the U.S. Food & Drug Administration (FDA) has accepted the filing of our New Drug Application (NDA) under the priority review for viltolarsen in patients with Duchenne Muscular Dystrophy (DMD) who are amenable to exon 53 skipping therapy. In addition to priority review, the FDA previously granted viltolarsen with Fast Track, Orphan Drug and Rare Disease designations.

The viltolarsen NDA includes results from a Phase 2 study and its long-term extension study in North America -- as well as a Phase 1 and a Phase 1/2 study in Japan. Both the Phase 1/2 and Phase 2 studies evaluated changes in dystrophin levels and motor function across two doses (40 mg/kg and 80 mg/kg weekly dose groups).

The PDUFA date for viltolarsen is within the 3rd quarter (July-September) of 2020. The PDUFA date is the target date the FDA provides a decision on the approval of a new drug.

Viltolarsen represents one of the most extensively studied antisense therapies in DMD. Viltolarsen, if approved by the FDA, would represent a new treatment option for DMD patients amenable to exon 53 skipping in the United States.

About Duchenne Muscular Dystrophy (DMD)

DMD is a progressive form of muscular dystrophy that occurs primarily in males. DMD causes progressive weakness and loss (atrophy) of skeletal, heart, and pulmonary muscles. Early signs of DMD may include delayed ability to sit, stand, or walk and difficulties learning to speak. DMD may also affect learning and memory, as well as communication and certain social emotional skills. Most children with DMD use a

wheelchair full-time by age 13. Heart and respiratory muscle problems begin in the teen years and lead to serious, life- threatening complications.

About Viltolarsen

Viltolarsen has been granted a Rare Pediatric Disease Designation, Orphan Drug Designation, and a Fast Track Designation in the U.S., and "SAKIGAKE designation", "Orphan drug designation", and designation of Conditional Early Approval System in Japan. Viltolarsen is not approved by any regulatory authority and its safety and effectiveness has not been established.

About NS Pharma, Inc.

NS Pharma, Inc., is a wholly owned subsidiary of Nippon Shinyaku Co., Ltd. For more information, please visit <http://www.nspharma.com>. NS Pharma is a registered trademark of the Nippon Shinyaku group of companies.

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