

NEWS RELEASE



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FDA Grants Rare Pediatric Disease Designation to NS-050/NCNP-03 for the Treatment of Duchenne Muscular Dystrophy

PARAMUS, NJ: September 10, 2024 – NS Pharma, Inc. (NS Pharma), a subsidiary of Nippon Shinyaku Co., Ltd. (Nippon Shinyaku), announced that the Food and Drug Administration (FDA) has granted rare pediatric disease designation to NS-050/NCNP-03 which is being developed for the treatment of Duchenne muscular dystrophy (Duchenne). The FDA's rare pediatric disease designation is granted for treatments of serious or life-threatening diseases that affect children under the age of 18 and fewer than 200,000 patients in the United States.

“The journey from first symptom to diagnosis and finally treatment can be long and challenging for patients with rare diseases and their caregivers,” said NS Pharma President Yukiteru Sugiyama, Ph.D. “We are grateful for this designation which can help us accelerate the development of this therapy for Duchenne.”

Duchenne is a progressive muscle wasting disease caused by a deficiency of the dystrophin protein. It leads to weakness of skeletal, cardiac and pulmonary muscles. There are many types of genetic mutations that can cause Duchenne, and NS-050/NCNP-03 is being developed to treat patients with confirmed gene mutations amenable to exon 50 skipping therapy.

NS-050/NCNP-03 is an antisense oligonucleotide co-discovered by the National Center of Neurology and Psychiatry (NCNP) and Nippon Shinyaku. NS-050/NCNP-03 skips part of the genetic information of the dystrophin gene and produces a functional dystrophin protein with a slightly shorter chain length, which is expected to have the effect of suppressing muscle function deterioration.

NS Pharma is working to develop products for patients with rare diseases. We are preparing a Phase 1 / 2 study to evaluate the safety and efficacy of NS-050/NCNP-03 in patients with Duchenne in Japan and the United States.

About Duchenne Muscular Dystrophy (Duchenne)

Duchenne is a progressive form of muscular dystrophy that occurs primarily in males. It causes progressive weakness and loss of skeletal, cardiac, and respiratory muscles. Early signs of Duchenne may include delayed ability to sit, stand or walk. There is a progressive loss of mobility, and by adolescence, patients with Duchenne may require the use of a wheelchair. Cardiac and respiratory muscle problems begin in the teenage years and lead to serious, life-threatening complications. For more information about Duchenne, please visit wespeakduchenne.com.

About NS Pharma, Inc.

NS Pharma, Inc., is a wholly owned subsidiary of Nippon Shinyaku Co., Ltd. NS Pharma is a registered trademark of the Nippon Shinyaku Co., Ltd. For more information, please visit nspharma.com.

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