

NEWS RELEASE



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FDA Grants Rare Pediatric Disease Designation to NS-051/NCNP-04 for the Treatment of Duchenne Muscular Dystrophy

PARAMUS, NJ: January 23, 2025 – NS Pharma, Inc. (NS Pharma), a subsidiary of Nippon Shinyaku Co., Ltd. (Nippon Shinyaku), announced that Food and Drug Administration (FDA) has granted rare pediatric disease designation to NS-051/NCNP-04 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.

Duchenne is a progressive muscle wasting disease caused by a deficiency of the dystrophin protein. It leads to weakness of skeletal, cardiac, and respiratory muscles. There are many types of genetic mutations that can cause Duchenne, and NS-051/NCNP-04 is being developed to treat patients with confirmed gene mutations amenable to exon 51 skipping therapy.

NS-051/NCNP-04 is an antisense oligonucleotide co-discovered by the National Center of Neurology and Psychiatry (NCNP) and Nippon Shinyaku. NS-051/NCNP-04 promotes skipping of exon 51 of the dystrophin gene that produces a shortened dystrophin protein containing essential functional portions, which is expected to have the effect of stabilizing or improving muscle function.

NS Pharma is working to develop products for patients with rare diseases.

About Duchenne Muscular Dystrophy (Duchenne)

Duchenne is a 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

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