

FDA Grants Orphan Drug Designation to NS-051/NCNP-04 for the Treatment of Duchenne Muscular Dystrophy

PARAMUS, NJ: September 19, 2025 – NS Pharma, Inc. announced today that the U.S. Food & Drug Administration (FDA) has granted Orphan Drug Designation to NS-051/NCNP-04 which is being developed for the treatment of Duchenne muscular dystrophy (Duchenne) in patients amenable to exon 51 skipping. The FDA issues Orphan Drug Designations to support the development and evaluation of new treatments to prevent, diagnose, or treat a rare disease or condition.

NS-051/NCNP-04 previously received Rare Pediatric Disease Designation from the FDA in January 2025. For more information, see the press release [here](#).

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 the skipping of exon 51 within the dystrophin gene, causing the production of a shortened dystrophin protein containing essential functional portions. This is expected to have the effect of stabilizing or improving muscle function.

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

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