NEWS RELEASE

NIPPON SHINYAKU CO., LTD.

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

Kyoto, Japan, September 19, 2025 - Nippon Shinyaku Co., Ltd. (Nippon Shinyaku; Headquarters, Kyoto; President, Toru Nakai) announced that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation to NS-051/NCNP-04 which is being developed for the treatment of Duchenne Muscular Dystrophy (DMD).

The Orphan Drug Designation in the U.S. is granted for treatments for diseases affecting fewer than 200,000 patients in the U.S., supporting their development and evaluation. The Orphan Drug Designation provides NS-051/NCNP-04 with seven years of exclusive marketing rights. NS-051/NCNP-04 has already been granted Rare Pediatric Disease Designation by the FDA in January 2025.

DMD 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 DMD, 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 National Center of Neurology and Psychiatry (NCNP, Kodaira City in Tokyo; President, Kazuyuki Nakagome) and Nippon Shinyaku. NS-051/NCNP-04 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.

At Nippon Shinyaku, we are committed to developing innovative new therapies for intractable and rare diseases with a sense of mission and actively working to bring new treatment options to DMD patients as soon as possible.

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