

NEWS RELEASE



January 23, 2025

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

Kyoto, Japan, January 23, 2025 - Nippon Shinyaku Co., Ltd. (Nippon Shinyaku; Headquarters, Kyoto; President, Toru Nakai) announced that the 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 (DMD).

The FDA's Rare Pediatric Disease Designation is granted for treatments intended for serious or life-threatening diseases that affect children under the age of 18 and less than 200,000 patients in the U.S.

DMD is a progressive muscle dystrophy caused by deficiency of the dystrophin protein leading to weakness of skeletal, cardiac and pulmonary muscles. There are many types of genetic mutations in DMD, and NS-051/NCNP-04 targets to treat DMD 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; 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.

Nippon Shinyaku has been working actively with a sense of mission to develop agents for the treatment of intractable and rare diseases, with a view to launching products for DMD patients as soon as possible.

Contact

Corporate Communications Dept., Nippon Shinyaku Co., Ltd.

e_mail_kouhou@po.nippon-shinyaku.co.jp