

NEWS RELEASE



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FDA Grants Orphan Drug Designation to NS-089/NCNP-02 for the Treatment of Duchenne Muscular Dystrophy

Kyoto, Japan, August 7, 2023 - Nippon Shinyaku Co., Ltd. (Nippon Shinyaku; Headquarters, Kyoto; President, Toru Nakai) announced today that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation to NS-089/NCNP-02 (brogidirsen) which is being developed for the treatment of Duchenne Muscular Dystrophy (DMD).

The Orphan Drug Designation in the United States is for rare diseases with less than 200,000 patients in the United States. The Orphan Drug Designation provides NS-089/NCNP-02 with seven years of exclusive marketing rights and tax incentives. NS-089/NCNP-02 has already been granted as Rare Pediatric Disease Designation in June and Breakthrough Therapy Designation in July by the FDA this year.

DMD is a progressive muscular dystrophy that causes weakness in skeletal, cardiac, and pulmonary muscles due to a deficiency in the dystrophin protein that supports muscle cells. There are various genetic mutations in DMD, and NS-089/NCNP-02 is targeted for DMD patients who have been confirmed to have a gene mutation that can be treated by exon 44 skipping.

NS-089/NCNP-02 is an antisense nucleic acid discovered through joint research between our company and the National Center of Neurology and Psychiatry (Kodaira City, President: Kazuyuki Nakagome). NS-089/NCNP-02 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.

A Phase II study of NS-089/NCNP-02 in the United States is scheduled to be conducted by our overseas subsidiary NS Pharma, Inc. (Headquarters: New Jersey, USA, President: Tsugio Tanaka). In Japan, we are also preparing Phase II study.

Nippon Shinyaku has been working actively having 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.

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