

NEWS RELEASE



NIPPON SHINYAKU CO., LTD.

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

Kyoto, Japan, December 21, 2023 - Nippon Shinyaku Co., Ltd. (Nippon Shinyaku; Headquarters, Kyoto; President, Toru Nakai) announced that the European Commission (EC) 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 by the EC is issued to drugs which are intended for diseases that affect fewer than five in 10,000 people in the European Union and are life-threatening or chronically debilitating. The Orphan Drug Designation provides NS-089/NCNP-02 with potential ten years of exclusive marketing rights and tax incentives. NS-089/NCNP-02 has already been granted as Rare Pediatric Disease Designation in June 2023, and Breakthrough Therapy Designation and Orphan Drug Designation in July 2023 by the U.S. Food and Drug Administration.

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.

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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