NEWS RELEASE

NIPPON SHINYAKU CO., LTD.

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Senkuteki Iyakuhin (Pioneering Drug) Designation and Orphan Drug Designation granted to NS-089/NCNP-02 for the Treatment of Duchenne Muscular Dystrophy

Kyoto, Japan, December 2, 2024 - Nippon Shinyaku Co., Ltd. (Nippon Shinyaku; Headquarters, Kyoto; President, Toru Nakai) announced today that the Minister of Health, Labor and Welfare (MHLW) has granted Senkuteki Iyakuhin (Pioneering Drug) Designation and Orphan Drug Designation to NS-089/NCNP-02 which is being developed for the treatment of Duchenne Muscular Dystrophy (DMD).

Senkuteki Iyakuhin Designation System aims to shorten the period of the pharmaceutical review process, so that patients can access innovative medicines earlier. The designated drug must meet all four of the following requirements: “the innovativeness of the therapeutic agent”, “the severity of the target disease”, “the extremely high efficacy of the agent for the target disease”, and “the requirement of having the intention and system to develop and apply the agent in Japan ahead of the rest of the world”. In addition, Orphan drug Designation is based on the fact that the number of patients in Japan is 50,000 or less and that there is a particularly high level of medical need. With these designations, the drug will be treated as a priority review item, in addition to be able to receive various support measures for development, etc.

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 for Psychiatry and Neurological Medicine (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 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.

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