NEWS RELEASE

NIPPON SHINYAKU CO., LTD.

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FDA Grants Rare Pediatric Disease Designation to NS-089/NCNP-02 for the Treatment of Duchenne Muscular Dystrophy

Kyoto, Japan, July 4, 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 Rare Pediatric Disease Designation to NS-089/NCNP-02 (brogidirsen) 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-089/NCNP-02 targets to treat DMD patients with confirmed gene mutations amenable to exon 44 skipping therapy.

NS-089/NCNP-02 is an antisense oligonucleotide co-discovered by National Center of Neurology and Psychiatry (NCNP, Kodaira City; President, Kazuyuki Nakagome) and Nippon Shinyaku. 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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