NEWS RELEASE

NIPPON SHINYAKU CO., LTD.

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NS-089/NCNP-02: Preclinical data published in Molecular Therapy Nucleic Acids

Kyoto, Japan, October 17, 2023 - Nippon Shinyaku Co., Ltd. (Nippon Shinyaku; Headquarters, Kyoto; President, Toru Nakai) announced the publication of preclinical data on NS-089/NCNP-02 (brogidirsen) which is being developed for the treatment of Duchenne muscular dystrophy (DMD) in the journal *Molecular Therapy Nucleic Acids*. The article, "Exon 44 skipping in Duchenne muscular dystrophy: NS-089/NCNP-02, a dual-targeting antisense oligonucleotide," is available under open access.
(https://www.sciencedirect.com/science/article/pii/S2162253123002524)

NS-089/NCNP-02 is a connected antisense oligonucleotide that target two separate sites within exon 44 of the dystrophin pre-mRNA sequence. The article describes the novel sequence design method used to create NS-089/NCNP-02 and the pharmacological effects of NS-089/NCNP-02 on cultured cells derived from DMD patients, and on cynomolgus monkeys as preclinical data. These data are based on the results of joint research between Nippon Shinyaku and the National Center of Neurology and Psychiatry (Kodaira City; President, Kazuyuki Nakagome).

Nippon Shinyaku and NS Pharma, Inc. (Paramus NJ, USA; President, Tsugio Tanaka), a wholly owned subsidiary of Nippon Shinyaku, are currently preparing for a Phase II study of NS-089/NCNP-02 in Japan and the United States, respectively. In the U.S., NS-089/NCNP-02 has received Rare Pediatric Disease Designation in June 2023, and Breakthrough Therapy Designation and Orphan Drug Designation in July 2023 from the Food and Drug Administration.

Nippon Shinyaku has been actively pursuing its mission to develop drugs for the treatment of intractable and rare diseases, and we aim to bring products to DMD patients as soon as possible.

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