NEWS RELEASE

NIPPON SHINYAKU CO., LTD.

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Brogidirsen (NS-089/NCNP-02) for the treatment of Duchenne muscular dystrophy：3.5-Year Clinical Trial Data Presented at the World Muscle Society 2025 Congress

Kyoto, Japan, October 14, 2025 - Nippon Shinyaku Co., Ltd. (Nippon Shinyaku; Headquarters, Kyoto; President, Toru Nakai) announced the efficacy and safety data of 3.5 years of administration based on an open-label extension study, including an investigator-initiated clinical trial of brogidirsen (NS-089/NCNP-02).
These data were presented as a poster by the National Center of Neurology and Psychiatry (NCNP, Kodaira City, Tokyo; President, Kazuyuki Nakagome) at the 30th annual International Congress of the World Muscle Society held in Austria from October 7 to 11, 2025.

【Title of the poster presentation】
3.5-year safety and efficacy data of Brogidirsen from DMD patients in open-label extension study

Brogidirsen is an antisense oligonucleotide co-discovered by Nippon Shinyaku and NCNP as an investigational therapy for DMD patients with dystrophin gene mutations that are amenable to exon 44 skipping.

The presented data are based on the investigator-initiated clinical trial conducted by NCNP and its extension study conducted by Nippon Shinyaku. These studies evaluated the efficacy and safety in 6 patients who received weekly intravenous administration of brogidirsen.
The results showed trends of high exon skipping efficiency and dystrophin expression level in biopsied muscles at week 25/26 and week 99/100. Furthermore, participants who remain ambulant after long-term brogidirsen administration maintained their motor function ability in assessment including the North Star Ambulatory Assessment. Safety evaluation results showed no serious or severe adverse events related to brogidirsen, or adverse events related to infusion-related reactions such as anaphylaxis, or discontinuation observed due to adverse events. The results suggest the potential of brogidirsen to slow the disease progression in patients with DMD who are amenable to exon 44 skipping.

This long-term extension trial is ongoing to investigate the efficacy and safety of longer-term administration. Further, a global Phase II study of brogidirsen is being conducted by Nippon Shinyaku and its subsidiary NS Pharma, Inc. (Headquarters: New Jersey, USA; President: Yukiteru Sugiyama).

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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