

October 1, 2026

Five-Year Clinical Trial Data for Brogirdisen (NS-089/NCNP-02), an Investigational Treatment for Duchenne Muscular Dystrophy, Presented at the World Muscle Society 2026 Congress

Kyoto, Japan, October 1, 2026 - Nippon Shinyaku Co., Ltd. (Nippon Shinyaku; Headquarters, Kyoto; President, Toru Nakai) announced the five-year efficacy and safety data for brogirdisen (NS-089/NCNP-02), obtained from an investigator-initiated clinical trial and its ongoing open-label extension study, were presented at the World Muscle Society 2026 Congress.

These data were presented in a poster by the National Center of Neurology and Psychiatry (NCNP, Kodaira City, Tokyo; President, Kazuyuki Nakagome) at the 31st annual International Congress of the World Muscle Society held in Hiroshima, Japan from September 29 to October 3, 2026.

【Poster presentation title】

Brogirdisen 5-Year Safety and Efficacy Outcomes in Duchenne Muscular Dystrophy Patients: Findings From an Ongoing Open-Label Extension Study

Brogirdisen 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 were obtained from an investigator-initiated clinical trial conducted by NCNP and its extension study conducted by Nippon Shinyaku. These studies evaluated the efficacy and safety of weekly intravenous administration of brogirdisen in six patients.

The efficacy results showed improvement or maintenance of motor function in participants who remained ambulant, including as assessed by the North Star Ambulatory Assessment (NSAA). Further, upper limb function was maintained in all participants including those who became non-ambulant during the course of the study. The safety results showed no serious or severe adverse events related to brogirdisen, no infusion-related reactions such as anaphylaxis, and no discontinuation due to adverse events. These findings suggest that brogirdisen has the potential to slow the disease progression in patients with DMD who are amenable to exon 44 skipping.

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

Nippon Shinyaku is committed to developing agents for the treatment of intractable and rare diseases, with a view to deliver new treatment options to patients with DMD as soon as possible.

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