

Brogidirsen (NS-089/NCNP-02) 4.5-Year Clinical Trial Data for the Treatment of Duchenne Muscular Dystrophy Presented at 2026 MDA Clinical & Scientific Conference

PARAMUS, NJ: March 9, 2026 – NS Pharma, Inc. (NS Pharma, New Jersey, USA; President, Yukiteru Sugiyama), a biopharmaceutical leader in rare diseases and subsidiary of Nippon Shinyaku Co., Ltd. (Nippon Shinyaku), announced today that the National Center of Neurology and Psychiatry (NCNP, Kodaira City; President, Kazuyuki Nakagome) presented 4.5-year safety and efficacy data based on the open-label extension study, including an investigator-initiated clinical trial of brogidirsen (NS-089/NCNP-02) for the treatment of Duchenne muscular dystrophy (DMD) at the 2026 MDA Clinical & Scientific Conference from March 8-11, 2026.

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.

“We are thrilled to see the data continue to demonstrate long-term efficacy in DMD patients amenable to exon 44 skipping and while maintaining patient safety,” said NS Pharma President, Yukiteru Sugiyama, Ph.D. “We are committed to expanding available treatment options to the DMD community and we look forward to realizing that ambition.”

The presented data are based on the first-in-human Phase 1/2 open-label investigator-initiated clinical trial conducted by NCNP and the subsequent Phase 2 open-label extension trial conducted by Nippon Shinyaku. These studies evaluate the safety and efficacy of brogidirsen in six participants who received weekly IV dosing of brogidirsen.

Findings include:

- **Maintenance of motor function** – From the initiation of weekly administration, participants maintained motor function in assessments such as North Star Ambulatory Assessment (NSAA). All participants, including those who transitioned to full-time wheelchair use due to disease progression, maintained their total scores in Performance of Upper Limb (PUL 2.0).
- **Acceptable safety profile** – After 4.5-year of receiving brogidirsen, no serious or severe adverse events were reported related to long-term brogidirsen administration; anaphylaxis was not observed and no patients discontinued administration.

These findings support the potential of brogidirsen to modify the progression of DMD. This extension trial is ongoing to investigate the safety and efficacy of longer-term administration.

A second, ongoing global Phase II study of brogidirsen is being conducted by Nippon Shinyaku and NS Pharma to further evaluate the safety and efficacy of brogidirsen. Learn more at ClinicalTrials.gov.

NEWS RELEASE



NS Pharma, Inc.
140 E. Ridgewood Ave. Suite 2805
Paramus, NJ 07652

About Duchenne Muscular Dystrophy (DMD)

Duchenne is a form of muscular dystrophy that occurs primarily in males. It causes progressive weakness and loss of skeletal, cardiac, and respiratory muscles. Early signs of DMD may include delayed ability to sit, stand or walk. There is a progressive loss of mobility, and by adolescence, patients with Duchenne may require the use of a wheelchair. Cardiac and respiratory muscle problems begin in the teenage years and lead to serious, life-threatening complications. For more information, please visit wespeakduchenne.com.

About NS Pharma, Inc.

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US Media Contact:

media@nspharma.com