



Paramus, NJ 07652

Brogidirsen (NS-089/NCNP-02) 3.5 Year Clinical Trial Data for the Treatment of Duchenne Muscular Dystrophy Presented at 2025 World Muscle Society Congress

PARAMUS, NJ: October 14, 2025 – NS Pharma, Inc. (NS Pharma), a biopharmaceutical leader in rare disease 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 3.5-year efficacy and safety data from the open-label extension of an investigator-initiated clinical trial of brogidirsen (NS-089/NCNP-02) for the treatment of Duchenne muscular dystrophy (DMD) at the 30th annual International Congress of the World Muscle Society from October 7 to 11, 2025.

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.

"These long-term motor function data suggest the potential for brogidirsen to slow disease progression in DMD patients amenable to exon 44 skipping and support the robust increases in dystrophin production seen earlier in the trial," said NS Pharma President, Yukiteru Sugiyama, Ph.D. "We are proud to offer hope to families and continue to demonstrate our commitment to the DMD community

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 of brogidirsen in 6 participants who received weekly IV dosing of brogidirsen.

Findings include:

- **Consistent functional benefits** High exon 44 skipping efficiency and dystrophin expression levels were observed in biopsied muscles at week 25/26 and week 99/100.
- **Maintenance of motor function** Participants who remain ambulant after long-term brogidirsen administration maintained their motor function in evaluations including North Star Ambulatory Assessment.
- **Acceptable safety profile** After 3.5-year of receiving brogidirsen, no serious or severe adverse events, or anaphylaxis related to long-term brogidirsen administration, were reported, and there were no discontinuations.

These findings support the potential of brogidirsen to modify the progression of DMD. 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 NS Pharma, Inc. (Headquarters: New Jersey, USA; President: Yukiteru Sugiyama). Learn more at <u>ClinicalTrials.gov</u>.

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





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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 <u>wespeakduchenne.com</u>.

About NS Pharma, Inc.

NS Pharma, Inc., is a wholly owned subsidiary of Nippon Shinyaku Co., Ltd. NS Pharma is a registered trademark of the Nippon Shinyaku Co., Ltd. For more information, please visit nspharma.com.

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