

NEWS RELEASE



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FDA Grants Breakthrough Therapy Designation to NS-089/NCNP-02 for the Treatment of Duchenne Muscular Dystrophy

NS Pharma, Inc. (NS Pharma; President, Tsugio Tanaka) is a wholly owned subsidiary of Nippon Shinyaku Co., Ltd. (Nippon Shinyaku; President, Toru Nakai)

PARAMUS, NJ: July 28, 2023 – NS Pharma, Inc. announced today the U.S. Food & Drug Administration (FDA) has granted Breakthrough Therapy Designation to NS-089/NCNP-02, an investigational candidate for patients with Duchenne muscular dystrophy amenable to exon 44 skipping therapy.

The Breakthrough Therapy Designation for NS-089/NCNP-02 is based on results from a first-in-human, investigator-initiated clinical trial conducted in Japan.¹ The FDA issues Breakthrough Therapy Designation to expedite the development and review of medicines which are intended to treat serious or life-threatening diseases. The criteria require preliminary clinical evidence that indicates the drug may demonstrate substantial improvement over available therapies on a clinically meaningful endpoint(s). In June 2023, NS-089/NCNP-02 was granted Rare Pediatric Disease Designation by the FDA.

NS-089/NCNP-02 is an antisense nucleotide discovered through joint research between NS Pharma's parent company, Nippon Shinyaku, and the National Center of Neurology and Psychiatry (Kodaira City, President: Kazuyuki Nakagome).

Clinical development of NS-089/NCNP-02 includes a planned Phase 2 study in the United States conducted by NS Pharma and a Phase 2 study conducted in Japan by Nippon Shinyaku. Additional details will be provided once the trials are ready to begin

enrolling participants.

About Duchenne Muscular Dystrophy (Duchenne)

Duchenne is a progressive form of muscular dystrophy that occurs primarily in males. Duchenne causes progressive weakness and loss of skeletal, cardiac, and respiratory muscles. Early signs of Duchenne 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.

About NS Pharma, Inc.

NS Pharma, Inc., is a wholly owned subsidiary of Nippon Shinyaku Co., Ltd. For more information, please visit <http://www.nspharma.com>. NS Pharma is a registered trademark of the Nippon Shinyaku group of companies.

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Reference

1. National Center of Neurology and Psychiatry Press release on March 17, 2022. <https://www.ncnp.go.jp/topics/2022/20220317e.html>