NEWS RELEASE

NIPPON SHINYAKU CO., LTD.

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

Kyoto, Japan, July 28, 2023 - Nippon Shinyaku Co., Ltd. (Nippon Shinyaku; Headquarters, Kyoto; President, Toru Nakai) announced today that the U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy Designation to NS-089/NCNP-02 (brogidirsen) which is being developed for the treatment of Duchenne Muscular Dystrophy (DMD).

The Breakthrough Therapy Designation is an FDA program designed to expedite the development and review of drugs for the treatment of serious or life-threatening conditions. Drugs that demonstrate significant preliminary clinical evidence on clinically important endpoints compared to existing treatments are eligible. This Breakthrough Therapy Designation is based on the results from Investigator-initiated Clinical Trial (First-in-human Trial) conducted in Japan.[1] In addition, NS-089/NCNP-02 has already been granted Rare Pediatric Disease Designation from the FDA in June of this year.

DMD is a progressive muscle dystrophy caused by deficiency of the dystrophin protein leading to weakness of skeletal, cardiac and pulmonary muscles. There are many types of genetic mutations in DMD, and NS-089/NCNP-02 targets to treat DMD patients with confirmed gene mutations amenable to exon 44 skipping therapy.

NS-089/NCNP-02 is an antisense oligonucleotide co-discovered by National Center of Neurology and Psychiatry (NCNP, Kodaira City; President, Kazuyuki Nakagome) and Nippon Shinyaku. NS-089/NCNP-02 skips part of the genetic information of the dystrophin gene and produces a functional dystrophin protein with a slightly shorter chain length, which is expected to have the effect of suppressing muscle function deterioration.

A Phase II study of NS-089/NCNP-02 in the United States is scheduled to be conducted by our overseas subsidiary NS Pharma, Inc. (Headquarters: New Jersey, USA, President: Tsugio Tanaka). In Japan, we are also preparing Phase II study.

Nippon Shinyaku has been working actively having 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.

Reference

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

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