NEWS RELEASE

NIPPON SHINYAKU CO., LTD.

April 14, 2023

Agreement with the U.S. FDA for a Global Phase II Study Plan of NS-089/NCNP-02 for the Treatment of Duchenne Muscular Dystrophy

Kyoto, Japan, April 14, 2023 - Nippon Shinyaku Co., Ltd. (Nippon Shinyaku; Headquarters, Kyoto; President, Toru Nakai) announced the U.S. Food and Drug Administration (FDA) has agreed to the planned global phase II study of NS-089/NCNP-02 for the treatment of Duchenne Muscular Dystrophy (DMD).

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.

The study will enroll patients with Duchenne muscular dystrophy who may benefit from exon 44 skip. Development in the United States will be carried out by our U.S. subsidiary NS Pharma, Inc. (Paramus NJ, USA; President, Tsugio Tanaka). We are also preparing clinical trials in Japan.

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 patients as soon as possible.

Contact
Corporate Communications Dept., Nippon Shinyaku Co., Ltd.
FAX: +81-75-321-9128