NEWS RELEASE

NIPPON SHINYAKU CO., LTD.

May 31, 2022

VILTEPSO® (viltolarsen): Long-Term Efficacy and Safety Data Published in the Journal of Neuromuscular Diseases

KYOTO, Japan, May 31, 2022 - Nippon Shinyaku Co., Ltd. (Nippon Shinyaku; Headquarters, Kyoto; President, Toru Nakai) announced the publication of long-term efficacy and safety data based on interim analyses at 109 weeks from the open-label extension trial of a Phase 2 study of VILTEPSO® (viltolarsen) in the Journal of Neuromuscular Diseases. The article, "Long-Term Functional Efficacy and Safety of Viltolarsen in Patients with Duchenne Muscular Dystrophy," is freely available under open access (https://content.iospress.com/articles/journal-of-neuromuscular-diseases/jnd220811).

Data published in the Journal of Neuromuscular Diseases are from an open-label trial (N=16) that is the extension of a previous 24-week Phase 2 trial in North America. All 16 patients aged 4 to <10 years with Duchenne Muscular Dystrophy (DMD) amenable to exon 53 skipping in the 24-week study elected to enroll in this long-term trial to continue evaluation of motor function and safety. Assessments of timed function tests (Time to Stand, Time to Run/Walk, 6-Minute Walk Test) were compared to a matched DMD historical control group (Cooperative International Neuromuscular Research Group Duchenne Natural History Study).

In addition to this Phase 2 open-label extension study, Nippon Shinyaku continues to investigate the efficacy and safety of VILTEPSO® in the confirmatory Phase 3 RACER53 trial.

About Duchenne muscular dystrophy (DMD)

DMD is a progressive muscular dystrophy that causes progressive weakness of skeletal, cardiac, and pulmonary muscles due to a gene mutation in the dystrophin gene. There are many types of genetic mutations that can cause DMD. VILTEPSO® is indicated for patients with DMD mutations that are amenable to exon 53 skipping.

About VILTEPSO ®

VILTEPSO® is a treatment for DMD in patients who have a confirmed mutation of the DMD gene that is amenable to exon 53 skipping. In Japan, this indication for the drug was approved by the Ministry of Health, Labour and Welfare (MHLW) under a conditional early approval system in March 2020. Nippon Shinyaku has sold it and conducted detailing activities for it since May the same year. In the United States, the drug received accelerated approval from the Food and Drug Administration (FDA) in August 2020 and has been marketed through NS Pharma, Inc.

About NS Pharma, Inc.

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

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