NEWS RELEASE

NIPPON SHINYAKU CO., LTD.

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VILTEPSO® (viltolarsen): Four-Year Clinical Trial Data Published in the Journal of Neuromuscular Diseases

KYOTO, Japan, May 9, 2023 - Nippon Shinyaku Co., Ltd. (Nippon Shinyaku; Headquarters, Kyoto; President, Toru Nakai) announced the publication of four-year efficacy and safety data from the open-label extension of a Phase 2 study of VILTEPSO® (viltolarsen) injection, for the treatment of Duchenne muscular dystrophy (DMD) in the Journal of Neuromuscular Diseases. The article, "Efficacy and Safety of Viltolarsen in Boys With Duchenne Muscular Dystrophy: Results From the Phase 2, Open-Label, 4-Year Extension Study," is freely available under open access. (https://content.iospress.com/articles/journal-of-neuromuscular-diseases/jnd221656)

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 10 meters, Time to Climb 4 Stairs) were compared to a matched DMD historical control group. Both groups received a stable dose of glucocorticoid treatment.

For efficacy at 205 weeks after treatment, the primary endpoint of mean change from baseline for Time to Stand was 2.7 seconds in the viltolarsen group, and 8.3 seconds in the historical control control group.

The most frequently reported adverse events in this study included cough, nasopharyngitis, rash, pyrexia, and vomiting, all of these were mild to moderate. This safety profile was similar to that seen in the previous short-term study, and there were no treatment-related serious adverse events and no treatment discontinuations.

Nippon Shinyaku will continue 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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