NEWS RELEASE

NIPPON SHINYAKU CO., LTD.

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VILTEPSO® (viltolarsen) injection: Four-Year Clinical Trial Data Presented at the World Muscle Society 2022 Conference

Kyoto, Japan, Oct 24, 2022 – Nippon Shinyaku Co., Ltd. (Nippon Shinyaku; Headquarters, Kyoto; President, Toru Nakai) announced today that 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), was presented at the World Muscle Society 2022 Conference held in Halifax, Canada from October 11 to 15.

The presented data are from an open-label trial that is the extension of a previous 24-week Phase 2 trial in North America. All 16 patients aged 4 to <10 years with DMD amenable to exon 53 skipping in the 24-week study were enrolled in this long-term trial to continue the 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 group-matched DMD historical control drawn from the Cooperative International Neuromuscular Research Group Duchenne Natural History Study (CINRG DNHS). 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 CINRG DNHS control group (p=0.0040). The secondary endpoints of mean change from baseline for Time to Run/Walk 10 meters was 2.0 seconds in the viltolarsen group and 6.0 seconds in the CINRG DNHS control group (p=0.0002), and mean change from baseline for Time to Climb 4 Stairs velocity was -0.01 m/s in the viltolarsen group and -0.13 m/s in the CINRG DNHS control group (p=0.0088).

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.

About VILTEPSO®

VILTEPSO® is indicated for the treatment of DMD patients who have a confirmed mutation of the DMD gene that is amenable to exon 53 skipping. VILTEPSO® received marketing authorization under an accelerated approval pathway in Japan in March 2020 and became commercially available in Japan in May of the same year.

In the United States, it received accelerated approval from the U.S. Food and Drug Administration (FDA) in August 2020 and has marketed through NS Pharma, Inc.

Nippon Shinyaku is conducting a Phase 3 randomized, double-blind, placebo-controlled trial (RACER53 trial) from October 2019. The purpose of this Phase 3 trial is to evaluate the efficacy on motor function and safety of viltolarsen compared to placebo in patients with DMD amenable to exon 53 skipping.

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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