NEWS RELEASE

May 9, 2023

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

Data were from a completed four-year study (up to Week 216) of the open-label extension trial of a VILTEPSO Phase 2 study

NS Pharma, Inc. (NS Pharma; President, Tsugio Tanaka), is a wholly owned subsidiary of Nippon Shinyaku Co., Ltd. (Nippon Shinyaku; President, Toru Nakai)

Paramus, NJ: May 9, 2023 - NS Pharma is pleased to announce that previously reported long-term efficacy and safety data (final visit up to Week 216) from the open-label extension of a Phase 2 study of VILTEPSO® (viltolarsen) have been published in the May issue of 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 (click here).

"In this extension study it was encouraging to see that, compared to the CINRG DNHS control group, Viltepso-treated patients showed maintenance of motor function through the first two years of treatment and experienced delay of disease progression over the following two years of treatment, whereas the control group declined over this same four-year time period," said Leslie Magnus, MD, Vice President, Medical Affairs. "NS Pharma is pleased that these data support the role of VILTEPSO as an important treatment strategy for DMD patients who are amenable to exon 53 skipping."

The 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 DMD amenable to exon 53 skipping at baseline 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, 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.

Efficacy results at Week 205 for VILTEPSO compared to the CINRG DNHS control group for the primary endpoint of mean change from baseline for Time to Stand was 2.7 seconds vs. 8.3 seconds, respectively.

The most frequently reported adverse events were mild to moderate and included cough, nasopharyngitis, rash, pyrexia, and vomiting. This safety profile was similar to that seen in the

previous short-term study. There were no treatment-related serious adverse events and no treatment discontinuations.

About VILTEPSO® (viltolarsen) injection

Prior to its approval in the U.S. in August 2020, VILTEPSO was granted Priority Review as well as Rare Pediatric Disease, Orphan Drug and Fast Track Designations. In March 2020, VILTEPSO was approved in Japan for the treatment of patients with DMD who are amenable to exon 53 skipping therapy. Prior to its approval in Japan, VILTEPSO was granted with the SAKIGAKE designation, Orphan Drug designation, and designation of Conditional Early Approval System.

Indication

VILTEPSO is indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the DMD gene that is amenable to exon 53 skipping. This indication is approved under accelerated approval based on an increase in dystrophin production in skeletal muscle observed in patients treated with VILTEPSO. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial.

Important Safety Information

Warnings and Precautions: Kidney toxicity was observed in animals who received viltolarsen. Although kidney toxicity was not observed in the clinical studies with VILTEPSO, the clinical experience with VILTEPSO is limited, and kidney toxicity, including potentially fatal glomerulonephritis, has been observed after administration of some antisense oligonucleotides. Kidney function should be monitored in patients taking VILTEPSO. Serum creatinine may not be a reliable measure of kidney function in DMD patients.

Serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio should be measured before starting VILTEPSO. Consider also measuring glomerular filtration rate before starting VILTEPSO. During treatment, monitor urine dipstick every month, and serum cystatin C and urine protein-to-creatinine ratio every three months.

Urine should be free of excreted VILTEPSO for monitoring of urine protein. Obtain urine either prior to VILTEPSO infusion, or at least 48 hours after the most recent infusion. Alternatively, use a laboratory test that does not use the reagent pyrogallol red, which has the potential to generate a false positive result due to cross reaction with any VILTEPSO in the urine. If a persistent increase in serum cystatin C or proteinuria is detected, refer to a pediatric nephrologist for further evaluation.

Adverse Reactions: The most common adverse reactions include upper respiratory tract infection, injection site reaction, cough, and pyrexia.

To report an adverse event, or for general inquiries, please call NS Pharma Medical Information at 1-866-NSPHARM (1-866-677-4276)

For more information about VILTEPSO, see full Prescribing Information.

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