NEWS RELEASE

May 31, 2022

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

Efficacy and safety results were based on analyses at week 109 from the open-label extension trial of a VILTEPSO Phase 2 study.

These data from the open-label extension study of VILTEPSO were previously presented at medical congresses and scientific meetings.

PARAMUS, NJ: May 31, 2022 – NS Pharma, Inc. (NS Pharma; President, Tsugio Tanaka), a wholly owned subsidiary of Nippon Shinyaku Co., Ltd. (Nippon Shinyaku; President, Toru Nakai), announced the publication of long-term efficacy and safety data based on analyses at 109 weeks from the 192-week open-label extension trial of a Phase 2 study of VILTEPSO® (viltolarsen) injection 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 (click here).

"In this VILTEPSO open-label, long-term extension study, evaluation of functional clinical endpoints demonstrated maintenance of motor function versus functional decline in a historical control group over two years." said Leslie Magnus, MD, Vice President, Medical Affairs. "These encouraging interim results with VILTEPSO support the continued need to research its clinical profile and its potential impact on maintaining mobility."

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 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, NS Pharma continues to investigate the efficacy and safety of VILTEPSO in the confirmatory Phase 3 RACER53 trial. This study was initiated in October 2019 and is currently enrolling patients. The purpose of this Phase 3 randomized, double-blind, placebo-controlled trial is to evaluate the efficacy of viltolarsen on functional motor endpoints compared to placebo in DMD patients amenable to exon 53 skipping. Continued approval of VILTEPSO is dependent on verification of clinical benefit.

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.

About NS Pharma, Inc.

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