NEWS RELEASE

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NS Pharma Reports on Results of VILTEPSO® (viltolarsen) injection After Four Years of Treatment in Open-Label Extension Trial in Duchenne Muscular Dystrophy

Results were from a final analysis (up to Week 216) of the open-label extension trial of a VILTEPSO Phase 2 study.

In the study, the primary endpoint of Time to Stand and secondary endpoints, including Time to Run/Walk and Time to Climb 4 Stairs, were evaluated in comparison to a group-matched DMD historical control.

PARAMUS, NJ: October 24, 2022 – NS Pharma, Inc. (NS Pharma; President, Tsugio Tanaka), a wholly owned subsidiary of Nippon Shinyaku Co., Ltd. (Nippon Shinyaku; President, Toru Nakai), announced today that long-term efficacy and safety data (final analysis up to Week 216) from the open-label extension of a Phase 2 study of VILTEPSO® (viltolarsen) injection were presented at the 27th International Hybrid Annual Congress of the World Muscle Society (WMS 2022) in Halifax, Nova Scotia, Canada.

“These data represent the longest clinical experience of an exon 53 skipping therapy for the treatment of Duchenne muscular dystrophy. In this four-year study, Viltepso-treated patients maintained motor function over the first two years of treatment and experienced significant delay of disease progression over the following two years, compared with the CINRG DNHS control group which declined over this same time period,” said Leslie Magnus, MD, Vice President, Medical Affairs. “These data are encouraging as NS Pharma continues to evaluate the effect of Viltepso on improving or

stabilizing motor function in a Phase 3 study."

Data presented at WMS 2022 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, 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 significantly favored viltolarsen compared to the CINRG DNHS control group for the primary endpoint of mean change from baseline for Time to Stand (viltolarsen 2.7 seconds vs. 8.3 seconds for CINRG DNHS, p=0.0040), and secondary endpoints including Time to Run/Walk 10 meters (viltolarsen 2.0 seconds vs. 6.0 seconds for CINRG DNHS, p=0.0002), and Time to Climb 4 Stairs (viltolarsen -0.01 m/s vs. -0.13 m/s for CINRG DNHS, p=0.0088).

The most frequently reported adverse events were mild to moderate in this 216-week open-label extension period 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.

"This long-term study gives physicians who treat patients with DMD important information about the long-term impact of VILTEPSO on ameliorating the disease course," said Paula Clemens, MD, from the University of Pittsburgh School of Medicine. "Further study is ongoing, but these four-year data give confidence that VILTEPSO can be considered an important part of the treatment strategy for patients with DMD whose dystrophin mutations are amenable to exon 53 skipping therapy."

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 patients with DMD amenable to exon 53 skipping.

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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