NEWS RELEASE

May 27, 2024

NS Pharma Shares Preliminary Results of Viltolarsen (NS-065 / NCNP-01) Phase 3 Clinical Trial (RACER53 Study)

PARAMUS, NJ: May 27, 2024 – NS Pharma, Inc. (NS Pharma), a subsidiary of Nippon Shinyaku Co., Ltd., announced today that it has received preliminary analysis results from the global Phase 3 clinical trial (RACER53 study, NCT04060199) of NS-065/NCNP-01 (generic name: viltolarsen).

Viltolarsen was approved by the United States (US) Food and Drug Administration (FDA) in 2020 under the brand name VILTEPSO® – for the treatment of Duchenne muscular dystrophy (Duchenne) in patients who have a confirmed mutation of the dystrophin gene that is amenable to exon 53 skipping – under the FDA accelerated approval pathway based on an increase in dystrophin production in skeletal muscle observed in treated patients. In the US, continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial.

The RACER53 Study is a randomized, double-blind, placebo-controlled, comparative study of 77 ambulatory boys with Duchenne. The study evaluated the efficacy and safety of an 80 mg/kg once weekly dosing of the treatment – versus placebo – for 48 weeks and was intended to serve as a confirmatory study.

The primary endpoint of the study was Time to Stand from Supine evaluated as velocity (rise/sec). The viltolarsen group showed a trend of increased velocity from baseline after treatment for 48 weeks. However, the placebo group also showed a trend of increased velocity, and there was no statistically significant difference between the viltolarsen group and the placebo group.

Preliminary safety results indicated that all adverse events that occurred under viltolarsen treatment were mild or moderate. There were no treatment emergent adverse events that led to discontinuation of the drug during the study.

“We are currently conducting further detailed data analyses and identifying factors that may have influenced the results (e.g. age, treatment period, and effect of

concomitant drugs including glucocorticoid therapy)," said NS Pharma President Tsugio Tanaka, MSc. "Considering the results of prior clinical studies, we have confidence that viltolarsen can be a beneficial treatment for amenable patients with Duchenne."

Specifically, in addition to the increase in dystrophin production in skeletal muscle that formed the basis of the FDA approval, a previously reported Phase 2, open-label, long-term extension study evaluated viltolarsen in 16 subjects between the ages of four and 10 with Duchenne amenable to exon 53 skipping. The study found that subjects receiving viltolarsen showed statistically significant improvements in the study's primary endpoint of mean change from baseline for Time to Stand at week 205 as compared to a historical control group that was matched for key factors. In this study, treatment emergent adverse events were primarily mild or moderate. No study participants discontinued the study drug due to adverse events.

NS Pharma is currently conducting further detailed data analyses, including post-hoc data analyses, and plans to work closely with regulatory authorities to determine how to proceed based on the results of this analysis and in the best interests of patients. The company will report on additional analyses and discussions with the regulatory authorities at a later date.

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 Duchenne who are amenable to exon 53 skipping therapy. Prior to its approval in Japan, VILTEPSO was granted 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 patients with Duchenne. 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 Duchenne Muscular Dystrophy (Duchenne)
Duchenne is a progressive form of muscular dystrophy that occurs primarily in males. It causes progressive weakness and loss of skeletal, cardiac, and respiratory muscles. Early signs of Duchenne may include delayed ability to sit, stand or walk. There is a progressive loss of mobility, and by adolescence, patients with Duchenne may require the use of a wheelchair. Cardiac and respiratory muscle problems begin in the teenage years and lead to serious, life-threatening complications. For more information about Duchenne, please visit wespeakduchenne.com.

About NS Pharma, Inc.
NS Pharma, Inc., is a wholly owned subsidiary of Nippon Shinyaku Co., Ltd. NS Pharma is a registered trademark of the Nippon Shinyaku Co., Ltd. For more information, please visit nspharma.com.

U.S. Media Contact:
media@nspharma.com