

NEWS RELEASE



March 19, 2023

VILTEPSO® (viltolarsen) injection Open Label Extension Clinical Trial Data Scheduled for Presentation at the MDA Clinical & Scientific Conference 2023

Data were from a final four-year analysis (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, Dr. Toru Nakai)

Paramus, NJ: March 19, 2023 - NS Pharma is pleased to announce participation in the Muscular Dystrophy Association's (MDA) Clinical & Scientific Congress 2023 being held in Dallas, Texas. The company will be presenting previously reported 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).

The presentation will be given by Edward Smith, MD, Duke University School of Medicine. For more information, please visit the MDA Congress website to view the abstract:

<https://www.mdaconference.org/abstract-library/results-of-a-4-year-viltolarsen-extension-study-of-functional-and-safety-outcomes/>

“There have been amazing scientific advances in the treatment of Duchenne, which is why it is important to keep the community informed about existing therapeutic options and the long-term evidence these treatments have generated,” said Dr. Smith. “The Muscular Dystrophy Association is one of the premier advocacy groups supporting the Duchenne community and I am pleased to take part in this year’s knowledge sharing at the organization’s Clinical & Scientific Congress on behalf of NS Pharma.”

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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