

NEWS RELEASE



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NS Pharma, Inc. Shares New VILTEPSO® (Viltolarsen) Data at the MDA Clinical & Scientific Conference 2024

Evidence of meaningful benefit in pulmonary function for patients with Duchenne muscular dystrophy will also be presented at the AAN 2024 Annual Meeting

PARAMUS, NJ: March 6, 2024 – NS Pharma, Inc. (NS Pharma) is excited to announce participation in the 2024 Muscular Dystrophy Association (MDA) Clinical & Scientific Conference in Orlando, Florida, March 3 – 6. The company presented a poster entitled “Pulmonary and motor function in ambulatory and non-ambulatory participants with Duchenne muscular dystrophy (Duchenne) treated with viltolarsen (VILTEPSO®)” which covers data from the Galactic53 trial demonstrating that the majority of participants receiving viltolarsen experienced meaningful benefit in pulmonary function, including percent predicted forced vital capacity (FVC%p).

“Galactic53 is the first trial with VILTEPSO to evaluate pulmonary function in participants with Duchenne,” explains NS Pharma Vice President Medical Affairs & Pharmacovigilance Leslie Magnus, MD, who also co-authored the poster. “Our team is encouraged by these results and will continue our research into treatments for rare disease.”

Galactic53 was a Phase 2, open-label, multicenter study of viltolarsen administered intravenously, 80mg/kg once weekly, in both ambulatory and non-ambulatory individuals with Duchenne who are amenable to exon 53 skipping therapy. The study also found that the upper limb motor function of participants was stabilized over 49 weeks in both ambulatory and non-ambulatory patients. Viltolarsen was well tolerated by participants, and the safety profile was consistent with previous reports.

View the poster online: <https://www.nspharma.com/events>. Additional data from

this study will also be presented at the American Academy of Neurology (AAN) 2024 Annual Meeting, April 13 – 18 in Denver, Colorado and online.

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 in patients who have a confirmed mutation of the Duchenne 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.

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