NEWS RELEASE

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NS Pharma's Galactic53 Trial Data Is Published in Scientific Reports

PARAMUS, NJ: October 15, 2024 – NS Pharma, Inc. (NS Pharma), a subsidiary of Nippon Shinyaku Co., Ltd. (Nippon Shinyaku), announced that the Galactic53 study of VILTEPSO® (generic name: viltolarsen) – a treatment for Duchenne muscular dystrophy (Duchenne) – has been published in the journal Scientific Reports. The paper, "Safety and efficacy of viltolarsen in ambulatory and nonambulatory males with Duchenne muscular dystrophy", contains data from an open-label multicenter study, which was the first to evaluate the effects of viltolarsen on pulmonary function in participants with Duchenne.

Ten ambulatory and ten nonambulatory participants ages eight years and older – with a confirmed deletion of the dystrophin gene that could be treated by exon 53 skipping – received 80 mg/kg of the drug intravenously once weekly for 48 weeks. Safety was evaluated as the primary endpoint, and pulmonary and motor function were evaluated as secondary efficacy endpoints. The pulmonary endpoints were compared to natural history data with matched patient backgrounds as a control group.

"We're encouraged by these results for patients with exon 53 skip-amenable mutations," explains NS Pharma Vice President Medical Affairs & Pharmacovigilance Leslie Magnus, MD, who co-authored the study. "We're especially glad to report positive data was seen in both nonambulatory and ambulatory patients."

All treatment-emergent adverse events were mild or moderate. Four were considered treatment-related, and no participants discontinued. The side effect profile seen in this study is consistent with that reported in previous studies, and the treatment was well tolerated.

Both ambulatory and nonambulatory participants receiving viltolarsen experienced improved pulmonary function with higher percent predicted forced vital capacity (FVC%p) and higher peak cough flow (PCF) at Week 49 compared with controls. Ninety (90)% (nine / ten) of nonambulatory participants receiving viltolarsen had an increase or stabilization in FVC%p from baseline, and 60% (six / ten) of participants maintained FVC%p values >50% at Week 49, which is the recommended threshold

for needing cough assist and nighttime ventilation interventions. For ambulatory participants treated with viltolarsen, 90% (nine / ten) of viltolarsen treated participants had an increase or stabilization in FVC%p from baseline, and all treated participants maintained FVC%p values >50% at Week 49.

Viltolarsen treated participants, including those who were nonambulant, also showed stabilized arm strength and mobility as assessed by performance of upper limb (PUL) scores over the 48-week treatment period.

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 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.

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