NEWS RELEASE

NIPPON SHINYAKU CO., LTD.

August 13, 2020

VILTEPSO™ (viltolarsen) Now Approved in the U.S.
for the Treatment of Duchenne Muscular Dystrophy

KYOTO, Japan August 13, 2020 – Nippon Shinyaku Co., Ltd. (Nippon Shinyaku; HQ, Kyoto; President, Shigenobu Maekawa) announced that the U.S. Food & Drug Administration (FDA) has approved VILTEPSO™ (viltolarsen) injection for patients with Duchenne muscular dystrophy (DMD) who are amenable to exon 53 skipping therapy.

DMD is a progressive muscle disease that primarily occurs in boys due to a genetic mutation in the dystrophin gene. This prevents the production of normal dystrophin resulting in reduced muscle strength/function that progressively continues to decline. There are many types of genetic mutations that can cause DMD. VILTEPSO™ is indicated for patients with DMD mutations that are amenable to exon 53 skipping.

A phase 2 clinical study was conducted in North America by NS Pharma, Inc. (Paramus, NJ; President, Tsugio Tanaka), a wholly owned subsidiary of Nippon Shinyaku. Viltolarsen was granted Rare Pediatric Disease Designation, Orphan Drug Designation, and Fast Track Designation in the US. The FDA reviewed the NDA under an accelerated approval pathway, based on data from the phase 2 study conducted in North America and the phase 1/2 study conducted in Japan. Viltolarsen also received Priority Review and has now received marketing authorization in the US. Currently, a phase 3 study (RACER53 study) is ongoing in approximately 30 centers worldwide as a confirmatory study.

In Japan, VILTEPSO was granted marketing authorization by the Ministry of Health, Labour and Welfare (MHLW) in March 2020 and launched by Nippon Shinyaku in May 2020.
Nippon Shinyaku continues to focus its research efforts on the treatment of DMD in Japan and the U.S. VILTEPSO will be made available in the U.S. and marketed by NS Pharma.

< Summary of VILTEPSO >

Brand Name	VILTEPSO™ (viltolarsen) injection
Generic Name	Viltolarsen
Date of FDA approval	August 12, 2020
Dosage Forms and Strengths	Injection 250 mg/5 mL (50 mg/mL) solution in a single-dose vial
Indications and Usage	VILTEPSO is an antisense oligonucleotide 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.
Dosage and administration	Recommended dosage of VILTEPSO is 80 mg/kg of body weight once weekly. Administer as an intravenous infusion over 60 minutes.

< Duchenne Muscular Dystrophy (DMD) >

DMD is a progressive form of muscular dystrophy that occurs primarily in males. DMD causes progressive weakness and loss of skeletal, cardiac, and pulmonary muscles. Early signs of DMD may include delayed ability to sit, stand or walk. There is a progressive loss of mobility, and by adolescence, patients with DMD may require the use of a wheelchair. Cardiac and respiratory muscle problems begin in the teenage years and lead to serious, life-threatening complications.

< VILTEPSO (general name: Viltolarsen) >

VILTEPSO is antisense oligonucleotide indicated for the treatment of DMD) in patients who have a confirmed mutation of the DMD gene that is amenable to exon 53 skipping, which was co-discovered by Nippon Shinyaku and National Center of Neurology and Psychiatry (Kodaira City, Tokyo; President, Hidehiro Mizusawa). VILTEPSO was granted SAKIGAKE designation, Orphan Drug Designation, and designation of Conditional Early Approval System in Japan.

< Phase 2 study (201 study) in the U.S. >

In this Phase 2 clinical study, that included patients aged four to less than 10 years of age (N=16), 100% of patients (8/8) showed an increase in dystrophin levels after treatment with VILTEPSO, and 88% of patients (7/8) showed dystrophin level of 3% or greater than normal for patients taking VILTEPSO 80 mg/kg/wk (n=8).[1,2] Overall, after 20-24 weeks of treatment a mean increase in dystrophin expression to nearly 6% of normal was observed with

VILTEPSO (80 mg/kg/week) versus 0.6% at baseline (primary endpoint).[1,2] The most common side effects of VILTEPSO included upper respiratory tract infection, injection site reaction, cough, and fever.

< References >

1. Viltepso [prescribing information]. Paramus, NJ: NS Pharma, Inc.; 2019.
2. Clemens PR, Rao VK, Connolly AM, et al; for the CINRG DNHS Investigators. Safety, tolerability, and efficacy of viltolarsen in boys with Duchenne muscular dystrophy amenable to exon 53 skipping: a phase 2 randomized clinical trial [published online ahead of print May 26, 2020. JAMA Neurology. doi:10.1001/jamaneurol.2020.1264.

< About NS Pharma, Inc. >

NS Pharma, Inc., is a wholly owned subsidiary of Nippon Shinyaku Co., Ltd. For more information, please visit https://www.nspharma.com. NS Pharma is a registered trademark of the Nippon Shinyaku group of companies.

< IMPORTANT SAFETY INFORMATION >

- In clinical studies, no patients experienced kidney toxicity during treatment with VILTEPSO. However, kidney toxicity from drugs like VILTEPSO may be possible. Your doctor may monitor the health of your kidneys before starting and during treatment with VILTEPSO.
- The most common side effects of VILTEPSO included upper respiratory tract infection, injection site reaction, cough and fever.

For more information about VILTEPSO, see Full Prescribing Information.

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