NEWS RELEASE

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NS Pharma Announces Publication of Clinical Trial Data for Viltolarsen in DMD Patients in *JAMA Neurology*

Viltolarsen is an exon skipping therapy being reviewed under the FDA Accelerated Approval pathway for the treatment of Duchenne muscular dystrophy in patients amenable to exon 53 skipping therapy

PARAMUS, NJ: May 26, 2020 – NS Pharma, Inc. (NS Pharma; President, Tsugio Tanaka), a wholly owned subsidiary of Nippon Shinyaku Co., Ltd. (Nippon Shinyaku; President Shigenobu Maekawa), announced today that *JAMA Neurology* has published results from a clinical trial of viltolarsen, an investigational agent being evaluated in Duchenne muscular dystrophy (DMD) patients who are amenable to exon 53 skipping therapy.

“In this study, 100% of patients were shown to have more dystrophin after treatment with viltolarsen and 88% achieved dystrophin levels of greater than 3%,” said lead study author and investigator Paula Clemens, MD, University of Pittsburgh School of Medicine. “These increases were seen in patients as young as four years of age and after six months or less of treatment, which underscores the impressive results seen in this study.”

This Phase 2, two-period, dose-finding study enrolled 16 DMD patients, from four to less than 10 years of age, who were amenable to exon 53 skipping therapy. Study participants were randomized to two doses of viltolarsen (40 mg/kg/wk and 80 mg/kg/wk) for 20 to 24 weeks. At the end of the study, treatment with viltolarsen was associated with statistically significant increases in mean dystrophin expression (40 mg/kg/wk: $p<0.001$; 80 mg/kg/wk $p=0.012$). Mean dystrophin levels of 5.7% and 5.9% were observed in

comparison to mean baseline levels of 0.3% and 0.6% in the 40 mg/kg/wk and 80 mg/kg/wk groups, respectively. Fourteen out of 16 patients (88%) reached dystrophin levels greater than 3%.

The most common treatment emergent adverse events occurring in greater than one patient were: cold, cough, nasal congestion, bruising, joint pain, diarrhea and vomiting. No serious adverse events were observed in the study.

“Lack of functional dystrophin is recognized as the singular underlying cause of the devastating impact of DMD,” said study author and investigator Vamshi Rao, MD, Ann & Robert H. Lurie Children’s Hospital of Chicago. “As a pediatric neurologist who specializes in the treatment of DMD, I am encouraged by the dystrophin increases observed in this study and the potential of viltolarsen to address the underlying cause of DMD.”

“This is one of the first studies of exon skipping therapies in DMD to generate rigorous data on the primary biomarker of dystrophin protein,” said study author Eric Hoffman, PhD, Associate Dean for Research and Professor of Pharmaceutical Sciences at Binghamton University. “Having assisted in the development of viltolarsen for many years, and decades more researching the genetics and genomics of DMD, I am pleased by the results of this study and the implications for families facing DMD.”

Viltolarsen has not yet been approved in the U.S. and its New Drug Application was recently granted Priority Review by the FDA with an anticipated action date in the third quarter of 2020. In March 2020, viltolarsen was approved in Japan for the treatment of DMD patients amenable to exon 53 skipping therapy.

“We are deeply committed to the development of viltolarsen and offering healthier futures to DMD patients and families,” said Tsugio Tanaka, President, NS Pharma, Inc. “Based on these results we are optimistic that, if it is approved, viltolarsen will become an important new treatment option for DMD patients and healthcare providers.”

NS Pharma continues to study the safety and efficacy of viltolarsen in the confirmatory Phase 3 RACER53 trial. This study was initiated in October 2019 and is currently enrolling. The purpose of this Phase 3 trial is to confirm the clinical findings that were submitted under the Accelerated Approval pathway.

About 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.

About Viltolarsen

Viltolarsen has been granted Rare Pediatric Disease, Orphan Drug and Fast Track Designations in the U.S. The viltolarsen New Drug Application was granted Priority Review by the FDA with an anticipated action date in the third quarter of 2020. In March 2020, viltolarsen 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, viltolarsen was granted with the SAKIGAKE designation, Orphan drug designation, and designation of Conditional Early Approval System.

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