NEWS RELEASE

NIPPON SHINYAKU CO., LTD.

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VILTEPSO® (viltolarsen) injection: Long-Term Efficacy and Safety Data Presented at the PPMD 2021 Virtual Annual Conference

Kyoto, Japan, July 1, 2021 – Nippon Shinyaku Co., LTD. (Nippon Shinyaku; Headquarters, Kyoto; President, Toru Nakai) announced that new long-term efficacy and safety data based on interim analyses at 109 weeks from the open-label extension trial of a Phase 2 study of VILTEPSO® (viltolarsen) injection were presented at the PPMD 2021 Annual Conference.

【Presentation Summary】

- Efficacy and safety results were based on interim analyses at week 109 from the open-label extension trial of a VILTEPSO Phase 2 study.
- The primary endpoint of Time to Stand showed a statistically significant benefit for multiple time points over two years of treatment with VILTEPSO in comparison to DMD matched historical control group.
- Additional secondary endpoints of motor function including Time to Run/Walk and 6-Minute Walk Test demonstrated consistent and statistically significant benefits in comparison to DMD historical controls.
- There were no treatment related serious adverse events and no patients discontinued treatment.

“These analyses showed that, after more than two years of treatment with VILTEPSO, patients maintained their motor function based on clinically relevant measurements while the DMD historic controls showed functional decline,” said Leslie Magnus, MD, Vice President, Medical Affairs, NS Pharma, Inc.

This current open-label trial (N=16) is the extension of a previous 24-week trial in North America. All 16 patients in the short-term study elected to enroll in this long-term trial. Participants were assessed at weeks 37, 49, 73 and 109 and will continue to be assessed until study completion. These interim analyses of the timed function tests were conducted for all participants who had received at least 109 weeks of total treatment vs. the matched DMD

historical control group (Cooperative International Neuromuscular Research Group – Duchenne Natural History Study (CINRG-DNHS)).

The efficacy results were for Time to Stand from supine (mean change from baseline (seconds) at weeks 73 and 109 for viltolarsen was 0.21 and 0.43 vs CINRG-DNHS: 3.6 and 4.3, $p<0.01$), Time to Run/Walk 10 meters (mean change from baseline (seconds) at weeks 49, 73, and 109 for viltolarsen was -0.8, -0.9, and -0.4 vs CINRG-DNHS: 0.5, 1.3, 1.3, $p<0.05$), and 6-Minute Walk Test (mean change from baseline (meters) at week 109 for viltolarsen was 0.9 vs CINRG-DNHS: -65.6, $p<0.05$).

The most frequently reported adverse events were mild to moderate in this time-period and included cough, nasopharyngitis, rash, pyrexia, and vomiting. This safety profile was similar to that seen in the previous short-term study. To date, there were no treatment-related serious adverse events and no treatment discontinuations. These data provide important information on the efficacy and safety of the long-term use of viltolarsen for the treatment of DMD patients who are amenable to exon 53 skipping.

“Duchenne is a progressive disease of functional deterioration,” said study investigator Paula Clemens, MD, University of Pittsburgh Medical Center. “More research is needed, but a disease-modifying therapy that could stabilize and delay the loss of muscle function is needed for families with Duchenne and the healthcare professionals who specialize in its treatment.”

In addition to this Phase 2 open-label extension study, Nippon Shinyaku continues to investigate the efficacy and safety of VILTEPSO in the confirmatory Phase 3 RACER53 trial. This study was initiated in October 2019 and is currently enrolling patients. The purpose of this Phase 3 randomized, double-blind, placebo-controlled trial is to evaluate the efficacy of viltolarsen on functional motor endpoints compared to placebo in DMD patients amenable to exon 53 skipping.

About VILTEPSO

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. VILTEPSO received marketing authorization under an accelerated approval pathway in Japan in March 2020 and became commercially available in Japan in May of the same year. In the United States, it received accelerated approval from the US Food and Drug Administration (FDA) in August 2020 and has marketed through NS Pharma, Inc.

About NS Pharma, Inc.

NS Pharma, Inc., is a wholly owned subsidiary of Nippon Shinyaku Co., Ltd. For more information, please visit http://www.nspharma.com.

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