NEWS RELEASE

Marking authorization in Japan of VILTEPSO® Intravenous Infusion 250 mg for the treatment of Duchenne muscular dystrophy

KYOTO, Japan Mar. 25, 2020 – Nippon Shinyaku Co., Ltd. (President, Shigenobu Maekawa) announced today that the Ministry of Health, Labour and Welfare (MHLW) has approved VILTEPSO® Intravenous Infusion 250 mg (viltolarsen, previously NS-065/NCNP-01) for the treatment of patients with Duchenne Muscular Dystrophy (DMD) who are amenable to exon 53 skipping therapy. This represents the first regulatory approval for viltolarsen in the world and the first approved DMD treatment other than steroids in Japan.

DMD is a progressive form of muscular dystrophy that occurs primarily in males. DMD causes progressive weakness and loss (atrophy) of skeletal, cardiac, and pulmonary muscles due to a deficiency of normal dystrophin, a protein involved in constructing the framework of muscle cells.

VILTEPSO® Intravenous Infusion 250 mg is a morpholino antisense oligonucleotide, which was co-discovered by Nippon Shinyaku and National Center of Neurology and Psychiatry (NCNP: Kodaira City, Tokyo; President, Hidehiro Mizusawa, Executive Director, Shin’ichi Takeda). It is designed to increase dystrophin production by binding to exon 53, an exon next to the dystrophin gene deletion which results in the translation of the mRNA into a shortened dystrophin protein that contains essential functional portions. In Japan, VILTEPSO® Intravenous Infusion 250 mg was granted SAKIGAKE designation of MHLW in October 2015, Orphan drug designation in August in 2019, and designation of Conditional Early Approval System in October in 2019. In the U.S, viltolarsen was granted a Rare Pediatric Disease Designation, Orphan Drug Designation, and a Fast Track Designation.

Viltolarsen has not yet been approved in the U.S.
Nippon Shinyaku continues to focus their efforts on developing treatments for intractable and rare diseases, including DMD.
Summary of VILTEPSO

<table>
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<tr>
<th>Brand Name</th>
<th>VILTEPSO® Intravenous Infusion 250 mg</th>
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<tr>
<td>Generic Name</td>
<td>Viltolarsen</td>
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<tr>
<td>Dosage Forms and Strengths</td>
<td>Viltolarsen 250 mg/5 mL (50mg/mL) in a single-dose vial</td>
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<td>Indications and Usage</td>
<td>DMD patients who have a confirmed mutation of the DMD gene that is amenable to exon 53 skipping</td>
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<td>Dosage and administration</td>
<td>80 mg/kg of viltolarsen is administered intravenously once a week over 1 hour</td>
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<Viltolarsen>
Nippon Shinyaku conducted a phase 1/2 study and submitted NDA in September 2019. The North America P2 clinical study was conducted by NS Pharma, Inc. (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 U.S. Its New Drug Application (NDA) was granted Priority Review by the FDA with an anticipated action date in the third quarter of 2020. While viltolarsen remains under review by the FDA, NS Pharma continues to study the efficacy and safety of viltolarsen in the Phase 3 RACER53 trial initiated. To meet our commitment to the FDA and the DMD community, we initiated our confirmatory Phase 3 trial shortly after the completion of our NDA submission to the FDA.

Below are designations in Japan only.
<SAKIGAKE designation> (Japanese version of Breakthrough Therapy designation)
The designation is to promote Research and Development in Japan aiming at early practical application for world’s first, domestically-produced and innovative pharmaceutical products for serious and life-threatening diseases. This is aiming to shorten reviewing time for approval, facilitating a prioritized consultation and review for regulatory approval.

<Orphan Disease designation>
Orphan drug is defined as the medicine of which medical needs are particularly high for a disease of which patient number is less than 50,000 in Japan.

<Conditional Early Approval System>
"Conditional Early Approval System" is a system to put highly useful and effective drugs for treating serious diseases into practical use as early as possible.
Approval of applicable pharmaceutical products should be granted under a condition requiring the applicant to conduct post-marketing surveys or other studies that are necessary to re-confirm the efficacy and safety of the product. The system must be subjected to the following conditions.

1. For severe diseases
2. Apparent improvement of medical care
3. Confirmatory clinical trials is difficult to conduct, or don’t have sufficient feasibility.
4. Confirmation of a certain degree of efficacy and safety through clinical trials other than confirmatory clinical trials.

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