NEWS RELEASE

NIPPON SHINYAKU CO., LTD.

May 20, 2020

Launch of VILTEPSO® (viltolarsen) Intravenous Infusion 250 mg for the treatment of Duchenne muscular dystrophy patients amenable to exon 53 skipping therapy in Japan

KYOTO, Japan May 20, 2020 – Nippon Shinyaku Co., Ltd. (President, Shigenobu Maekawa, "Nippon Shinyaku") announced today that 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 is now available for prescription in Japan.

VILTEPSO® is a morpholino antisense oligonucleotide and, it received marketing authorization from the Ministry of Health, Labour and Welfare (MHLW) in March 25 and its National Health Insurance (NHI) price was listed today. The global phase 3 study is ongoing.

Summary of VILTEPSO®

Brand Name	VILTEPSO® Intravenous Infusion 250 mg
Generic Name	Viltolarsen
Date of approval	March 25, 2020
Date of NHI price listing	May 20, 2020
Date of launch	May 20,2020
Dosage forms and strengths	Viltolarsen 250 mg/5 mL (50mg/mL)
Indications and usage	DMD patients who have a confirmed mutation of the DMD gene that is amenable to exon 53 skipping
Dosage and administration	80 mg/kg of viltolarsen is administered intravenously once a week over 1 hour
Drug price	91,136 JPY / Vial (250mg/5mL)
Package	250mg/5mL in a single dose vial

Nippon Shinyaku will contribute to the treatment of patients with DMD by appropriately delivering medical information on VILTEPSO® to medical facilities.
VILTEPSO® has not yet been approved in the U.S.

< 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®>

VILTEPSO® was an oligonucleotide discovered by Nippon Shinyaku and National Center of Neurology and Psychiatry (NCNP: Kodaira City, Tokyo; President, Hidehiro Mizusawa). It is designed to produce a shortened dystrophin protein that contains essential functional portions in patients with DMD mutations amenable to exon 53 skipping by skipping exon 53 in the pre-messenger RNA of the DMD gene. After completion of a phase 1/2 study in Japan, NDA was submitted to in September, 2019. VILTEPSO® has received marketing authorization under an accelerated approval pathway in Japan in March 2020.

Contact (Japan):

Corporate Communications Dept., Nippon Shinyaku Co., Ltd.

FAX: +81-75-321-9128