

May 12, 2020

First Patient Enrolled in Phase 3 Trial of Viltolarsen (NS-065/NCNP-01), an Investigational Antisense Oligonucleotide

KYOTO, Japan: May 12, 2020 - Nippon Shinyaku Co., Ltd. (Nippon Shinyaku; President, Shigenobu Maekawa) announced today that NS Pharma (Paramus NJ, USA; President, Tsugio Tanaka), a wholly owned subsidiary of Nippon Shinyaku, has enrolled the first patient in a Phase 3 trial of viltolarsen in Japan.

This Phase 3 trial is a randomized, double-blind, placebo-controlled, global study including Japan and the U.S., assessing the safety and efficacy of viltolarsen in ambulant boys with Duchenne muscular dystrophy (DMD) who are amenable to exon 53 skipping therapy. In this trial, patients will receive once weekly intravenous (IV) infusions of viltolarsen 80 mg/kg or placebo for 48 weeks. The study has a planned enrollment of 74 boys with DMD who are 4 to less than 8 years old. Clinical efficacy will be assessed at regularly scheduled study visits, and will include functional tests* such as Time to Stand Test (TTSTAND), the primary endpoint. Additional functional tests will also be assessed as the secondary endpoints. Safety will be assessed through the collection of adverse events (AEs), laboratory tests, electrocardiograms (ECGs), vital signs, and physical examinations throughout the study. Additional information about this Phase 3 trial (NCT04060199) can be found on www.clinicaltrials.gov.

The overall clinical development of viltolarsen is characterized by our unwavering commitment to conduct robust trials that will help demonstrate the potential impact of viltolarsen on patients with this devastating disease.

In March 2020, viltolarsen was approved in Japan for the treatment of DMD patients amenable to exon 53 skipping therapy. 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.

Functional Tests*

- Time to Stand Test (TTSTAND)
- Time to Run/Walk 10 Meters Test (TTRW)
- Six-minute Walk Test (6MWT)
- North Star Ambulatory Assessment (NSAA)
- Time to Climb 4 Steps Test (TTCLIMB)
- Hand-held dynamometer (elbow extension, elbow flexion, knee extension and knee flexion on the dominant side only).

About Duchenne Muscular Dystrophy (DMD)

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. Early signs of DMD may include delayed ability to sit, stand, or walk and difficulties learning to speak. DMD may also affect learning and memory, as well as communication and certain social emotional skills. By adolescence, patients with DMD may require the use of a wheelchair. Cardiac and respiratory muscle problems begin in the teen years and lead to serious, life-threatening complications.

About Viltolarsen

Viltolarsen has been granted a Rare Pediatric Disease Designation, Orphan Drug Designation, and a Fast Track Designation in the U.S. Viltolarsen has received marketing authorization under an accelerated approval pathway in Japan in March 2020.

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>. NS Pharma is a registered trademark of the Nippon Shinyaku group of companies.

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