NEWS RELEASE

NIPPON SHINYAKU CO., LTD.

March 6, 2024

VILTEPSO® (viltolarsen) injection:
Phase II trial (Galactic53 trial) Data presented
at the 2024 Muscular Dystrophy Association Clinical & Scientific Conference

Kyoto, Japan, March 6, 2024 – Nippon Shinyaku Co., LTD. (Nippon Shinyaku; Headquarters, Kyoto; President, Toru Nakai) announced that the results of the Phase II trial (Galactic53 trial) of VILTEPSO® (viltolarsen) injection, for the treatment of Duchenne muscular dystrophy (DMD), were presented as a poster presentation at the 2024 Muscular Dystrophy Association Clinical & Scientific Conference held in Florida from March 3-6 (U.S. time).

【Title of the poster presentation】
Pulmonary and motor function in ambulatory and non-ambulatory participants with Duchenne muscular dystrophy treated with viltolarsen

Galactic53 was a Phase II, open-label, multicenter study of VILTEPSO®, and for the first time evaluated the effect on non-ambulatory DMD patients and respiratory function. VILTEPSO® was intravenously administered 80mg/kg once weekly for 48 weeks to 10 ambulatory and 10 non-ambulatory DMD patients aged 8 years and older who had a confirmed dystrophin gene deletion that could be treated by exon 53 skipping therapy.

The primary endpoint was safety, and the secondary endpoints were efficacy in respiratory function and motor function. In addition, for some efficacy items, comparisons were made with natural history data matched for patient background as a control group.

Regarding safety, drug-related treatment-emergent adverse events were hematuria (2 cases), allergic reaction and hypertension (1 case each), all of which were non-serious. There were no cases of treatment discontinuation due to adverse events, confirming that this drug was well tolerated. Regarding efficacy, upper limb function was maintained over 49 weeks in both ambulatory and non-ambulatory groups. Mean change from baseline of percent predicted forced vital capacity (FVC%p) was 5.2 ± 2.3% in the VILTEPSO® -treated group compared to -0.9 ± 1.5% in the natural history group, indicating a significant improvement in respiratory function with VILTEPSO® ($p=0.03$).

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 the conditional early approval system 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 been 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 https://www.nspharma.com. NS Pharma is a registered trademark of the Nippon Shinyaku group of companies.

Contact

Corporate Communications Dept., Nippon Shinyaku Co., Ltd.
FAX: +81-75-321-9128