NEWS RELEASE

NIPPON SHINYAKU CO., LTD.

October 11, 2024

VILTEPSO® (viltolarsen) injection:
Phase II study (Galactic53 trial) Data Published in Scientific Reports

Kyoto, Japan, October 11, 2024 - Nippon Shinyaku Co., Ltd. (Nippon Shinyaku; Headquarters, Kyoto; President, Toru Nakai) announced that the results of the Phase II study (Galactic53 trial) of VILTEPSO® (viltolarsen) injection, for the treatment of Duchenne muscular dystrophy (DMD) has been published in the journal Scientific Reports. The paper, "Safety and efficacy of viltolarsen in ambulatory and nonambulatory males with Duchenne muscular dystrophy" is freely available under open access.
https://www.nature.com/articles/s41598-024-70783-y

Data published in the Scientific Reports are from an open-label multicenter study, which was the first to evaluate the effects of viltolarsen on pulmonary function in participants with Duchenne.

Ten ambulatory and ten nonambulatory participants ages eight years and older – with a confirmed deletion of the dystrophin gene that could be treated by exon 53 skipping – received 80 mg/kg of viltolarsen intravenously once weekly for 48 weeks. Safety was evaluated as the primary endpoint, and pulmonary and motor function were evaluated as secondary efficacy endpoints. The pulmonary endpoints were compared to natural history data with matched patient backgrounds as a control group.

All treatment-emergent adverse events were mild or moderate. Four were considered treatment-related, and no participants discontinued. The safety profile seen in Galactic53 trial is consistent with that reported in previous studies.

In both ambulatory and nonambulatory participants receiving viltolarsen, the improvement in pulmonary function was suggested with higher percent predicted forced vital capacity (FVC%p) and higher peak cough flow (PCF) at Week 49 compared with controls.

For ambulatory participants treated with viltolarsen, 90% (nine / ten) of viltolarsen treated participants had an increase or stabilization in FVC%p from baseline, and all treated participants maintained FVC%p values >50% at Week 49. For nonambulatory participants, 90% (nine / ten) participants receiving viltolarsen had an increase or stabilization in FVC%p from baseline, and 60% (six / ten) of participants maintained FVC%p values >50% at Week 49. When FVC%p is <50%, it is recommended to begin cough assist and nocturnal noninvasive ventilation.

Upper limb motor function, as assessed by the Performance Upper Limb (PUL2.0) scores, was maintained throughout the treatment period in both ambulatory and nonambulatory participants treated with viltolarsen.

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