NEWS RELEASE

NIPPON SHINYAKU CO., LTD.

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Viltolarsen (NS-065/NCNP-01) for the treatment of Duchenne Muscular Dystrophy
Preliminary Results of the Analysis of the Phase III Trial (RACER53 Study)

Kyoto, Japan, May 27, 2024 - Nippon Shinyaku Co., Ltd. (Nippon Shinyaku; Headquarters, Kyoto; President, Toru Nakai) announced the preliminary analysis results from the global Phase III trial (RACER53 study, NCT04060199) of NS-065/NCNP-01 (generic name: Viltolarsen).

This study is a randomized, double-blind, placebo-controlled, multi-center, comparative study of 77 boys with ambulatory Duchenne muscular dystrophy (DMD) with dystrophin gene mutations that are amenable to exon 53 skipping. The study evaluated the efficacy and safety of viltlarsen against placebo by administering viltolarsen 80 mg/kg or placebo once weekly for 48 weeks.

The primary endpoint of the study was Time to Stand from Supine evaluated as velocity (rise/sec). The viltolarsen group showed a trend of increased velocity from baseline after treatment for 48 weeks. However, the placebo group also showed a trend of increased velocity, and there was no statistically significant difference between the viltolarsen group and the placebo group.

Regarding safety, there was no difference in the incidence of adverse events between the viltolarsen group and the placebo group. All adverse events occurred in the viltolarsen group were mild or moderate, and there were no cases that led to discontinuation of treatment.

We have a confidence that viltolarsen is a beneficial medicine in response to the need of DMD patients, considering the results of prior clinical studies*. We are currently conducting further detailed data analyses and identifying factors that may have influenced the results (e.g. age, treatment period, and effect of concomitant drugs including glucocorticoid therapy). We will work closely with regulatory authorities to determine how to proceed based on the results of the analysis and in the best interest of patients. We will report on additional analyses and discussions with the regulatory authorities at a later date.

* Results of prior clinical studies:
In addition to the increase of dystrophin protein production in skeletal muscles, the efficacy of viltolarsen in 16 patients aged 4 to <10 years with DMD in an open-label Phase II extension study has been published. At 205 weeks after treatment, viltolarsen-treated patients showed a statistically significant improvement in the primary endpoint of mean change from baseline for Time to Stand from Supine compared to a DMD historical control group that matched for key factors. All adverse events occurred in viltolarsen treatment were mild or moderate, and there were no cases that led to discontinuation of treatment.

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